Optimisation du fonctionnement du Fonds Spécial de Solidarité

KCE reports 133B

Centre fédéral d'expertise des soins de santé
Federaal Kenniscentrum voor de Gezondheidszorg
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Titre : Optimisation du fonctionnement du Fonds Spécial de Solidarité

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Disclaimer : Les experts externes ont été consultés sur une version (préliminaire) du rapport scientifique. Une version (finale) a ensuite été soumise aux validateurs. La validation du rapport résulte d'un consensus ou d'un vote majoritaire entre les validateurs. Ce rapport a été approuvé à l'unanimité par le Conseil d'administration Le KCE reste seul responsable des erreurs ou omissions qui pourraient subsister de même que des recommandations faites aux autorités publiques.

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PREFACE

Le Fonds Spécial de Solidarité (FSS) a été créé en 1990 dans le but d'assurer un filet de sécurité permettant d'éviter que des personnes puissent manquer de soins indispensables qui, bien que très chers, ne sont pas remboursés par l'assurance maladie obligatoire. La structure, le fonctionnement, un nombre limité de catégories d'intervention et les critères de recevabilité qui s'y rapportent, ont été définis par la loi. Avec le temps, les domaines d'intervention du FSS se sont progressivement élargis.

Après une petite vingtaine d'années de fonctionnement, il était bon et logique de se poser la question de savoir si le FSS avait atteint les objectifs pour lesquels il avait été créé et plus particulièrement si certains besoins n'étaient pas rencontrés. Pour des raisons méthodologiques, le KCE a estimé ne pas pouvoir répondre directement à cette question. Par contre, il semblait possible et utile d'évaluer les processus de fonctionnement du FSS et d'en tirer des enseignements pour formuler des propositions d'amélioration de ceux-ci. C'est ce qui a été fait dans ce rapport que l'on ne peut pas qualifier de purement scientifique ou empirique mais dont nous espérons que les observations, les réflexions plus théoriques et les suggestions d'alternatives permettront de mieux rencontrer les objectifs d'un tel filet de sécurité.

Nous remercions les experts et les parties prenantes pour leur collaboration diligente et leurs apports précieux et très intéressants dans cette problématique aigüe.

Jean Pierre CLOSON
Directeur général adjoint

Raf MERTENS
Directeur général

Résumé

OBJECTIFS DE L'ETUDE

L'objectif de la présente étude était d'évaluer le fonctionnement du Fonds Spécial de Solidarité (FSS) et de formuler des suggestions visant à en optimiser les procédures de travail.

L'étude a été divisée en trois volets :

- Description du fonctionnement du FSS en se focalisant sur son organisation, ses procédures et ses activités ;
- Evaluation du fonctionnement du FSS, en se concentrant sur les critères d'éligibilité et leur interprétation pour le remboursement des dépenses, la clarté de ces critères pour les parties intéressées, les canaux d'information via lesquels les parties prenantes ont connaissance du FSS, l'évaluation des procédures et des processus décisionnels ;
- Etude de filets de sécurité (comparables) en France, en Espagne et aux Pays-Bas, en ciblant les enseignements potentiels à en tirer pour la Belgique.

METHODES

Les informations relatives à l'organisation du FSS, ses procédures et ses activités ont été obtenues par le biais d'une approche à la fois quantitative et qualitative. Des entretiens ont été organisés avec certains agents de l'Institut National d'Assurance Maladie-Invalidité (INAMI), avec des membres du Collège des Médecins-Directeurs (l'organe de prise de décision au niveau du FSS) et avec le médecin dirigeant du FSS. En outre, un échantillon de données administratives extraites de la base de données du FSS a été analysé. Cet échantillon n'était pas aléatoire car il ne contenait que les données relatives aux patients ayant donné leur accord pour la consultation de leur dossier. Enfin, les publications du FSS ont été étudiées.

Pour l'évaluation du fonctionnement du FSS, des entretiens ont été réalisées auprès d'un échantillon de médecins prescripteurs, de services sociaux et d'associations de patients, de Pharma.be (l'organisation qui chapeaute l'industrie pharmaceutique en Belgique) et de représentants de l'industrie pharmaceutique, membres du groupe de travail sur les médicaments orphelins. Seules les parties prenantes ayant une expérience suffisante du FSS ont été interrogées.

Pour le volet international, des experts des différents pays sélectionnés ont été contactés. En outre, des cas typiques du FSS ont été utilisés dans le but de comparer la manière dont ils auraient été gérés en France, aux Pays-Bas et en Espagne.

CATEGORIES DE REMBOURSEMENT ET CRITÈRES D'ÉLIGIBILITÉ

Les principales catégories remboursables sont les frais de traitement médical associés à :

- des indications rares ;
- des maladies rares exigeant un traitement physiopathologique spécifique ;
- des maladies rares exigeant un traitement continu et complexe ;
- des techniques de traitement innovantes ;
- des maladies chroniques chez l'enfant ;
- des traitements médicaux à l'étranger ;

Dans chacune de ces catégories de remboursement, divers critères d'éligibilité doivent être satisfaits. Même s'il existe des critères d'éligibilité spécifiques à chaque catégorie, applicables de manière cumulative, les critères suivants reviennent fréquemment :

- la prestation doit être chère ;
- l'affection doit porter atteinte aux fonctions vitales du patient ;
- la prestation doit présenter une valeur scientifique et une efficacité reconnues;
- l'assurance soins de santé obligatoire ne propose aucune alternative acceptable;
- la prestation doit être prescrite par un médecin spécialisé dans le traitement de la maladie.

Certaines dépenses, notamment les suppléments d'honoraires, les suppléments de prix (par exemple des suppléments sur du matériel médical), les suppléments sur la chambre qui peuvent être comptés au patient (sur base de l'article 90 de la loi sur les hôpitaux), les tickets modérateurs et les frais de confort sont expressément exclus du remboursement. Toutefois, lorsqu'il s'agit du remboursement des dépenses liées au traitement médical d'enfants atteints d'une maladie chronique et âgés de moins de 19 ans, le FSS peut rembourser les quotes-parts personnelles qui n'ont pas été prises en considération dans le plafond du maximum à facturer (MAF). Idem pour la marge de délivrance et la marge de sécurité pour les implants coûteux. En outre, le FSS ne rembourse pas les frais liés à un traitement médical si ces frais sont éligibles pour un remboursement par d'autres assurances (privées) ou systèmes de remboursement.

PROCEDURE DE DEMANDE

Les demandes de remboursement de frais médicaux par le FSS sont introduites par chaque patient auprès de l'agence locale de sa mutualité qui transfère la demande à l'organisme assureur (échelon national). De là, la demande est relayée au FSS. A chacun de ces niveaux, la demande fait l'objet d'un contrôle administratif (pour vérifier si tous les documents requis sont présents) et médical (pour s'assurer que la demande répond aux critères médicaux de remboursement par le FSS). Au niveau du FSS, les décisions sont le plus souvent prises par le Collège des médecins-directeurs qui est constitué des médecins-directeurs (ou de leurs représentants) de chaque organisme assureur et de médecins de l'INAMI (dont notamment un représentant de la Commission de Remboursement des Médicaments (CRM)). Pour certains types de demandes, essentiellement des renouvellements, la décision peut être déléguée à un seul membre du collège. S'agissant du remboursement des frais liés à l'Epidermolyse Bulleuse congénitale, la prise de décision est déléguée aux mutualités. Les recours contre les décisions du FSS sont traités par les Tribunaux du travail. Le nombre de cas enregistrés dont ont été saisis les Tribunaux du travail a diminué de 100 en 2004 à 23 en 2008. Les jugements des Tribunaux du travail ne sont pas utilisés par le FSS comme directive ni comme jurisprudence pour les décisions futures relatives à des cas similaires. Cette situation n'est pas surprenante puisqu'il n'y a pas de continuité ni d'uniformité au niveau de l'interprétation de certains critères d'éligibilité par les tribunaux.

PROFILS DE DEPENSES DU FSS

Comme le montre le tableau ci-dessous, les frais médicaux représentent l'essentiel des montants globaux octroyés par le FSS. Ce sont les dépenses pour les médicaments qui se taillent la part du lion dans les débours du FSS. Le budget du FSS étant limité, le Fonds peut restreindre provisoirement le remboursement des frais à un certain pourcentage du total. En général, le pourcentage utilisé est de 60% ou 75%. Lorsqu'un remboursement de 75% est accordé, la quote-part totale du patient est limitée à une somme qui varie entre 1.000 € et 1.500 € sur base annuelle.

Un remboursement restreint (60%) du FSS est également possible dans certains cas problématiques spécifiques, lorsqu'aucun accord n'est trouvé entre l'entreprise et le SPF Economie quant au prix du médicament (exemple: Flolan), dans les cas où un médicament est utilisé de manière non indiquée sur la notice… Lorsque le remboursement du FSS n'est que de 60%, la quote-part du patient n'est pas plafonnée.

Le FSS a la faculté, après la fixation définitive du prix, d'accorder un remboursement complémentaire. Cette restriction temporaire rend incertain le remboursement total des frais du patient avec à la clé, des difficultés dans le chef du médecin prescripteur pour faire accepter le risque potentiel par l'hôpital (qui va avancer les fonds).

Tableau 1 : Total des montants octroyés

EUR	2003	2004	2005	2006	2007	2008
medical costs (§ 2+EB (3) +art. 25bis, tar § 1, tar § 2, quater)	14.169.225	14.944.229	6.977.852	8.969.777	9.853.777	7.086.419
travel costs (§ 4+ art. 25sexies)	19.107	20.593	12.593	6.588	9.671	10.295
costs of stay (§ 4+ art. 25sexies)	2.757	7.585	4.664	15.023	6.419	6.593
medical costs (§ 4+ art. 25sexies)	45.889	275.000	4.338	27.790	37.164	430
« chronically ill children » (§ 3 en artikel 25quinquies)	0	4.833	94.272	329.039	525.626	952.708
subtotal	14.236.978	15.252.240	7.093.719	9.348.217	10.432.657	8.056.445
Costs rulings labor courts	-2	-2	90.808	162.760	112.533	427.286
Total amount spent	14.236.978	15.252.240	7.184.528	9.510.977	10.545.190	8.483.731
Decisions delegated to the health insurance funds			288.741	797.118	660.461	342.278

Source: Rapports annuels du Fonds Spécial de Solidarité pour 2003, 2004, 2005, 2006, 2007, 2008

EVALUATION DU FONCTIONNEMENT

INTERPRETATION DES CRITÈRES D'ÉLIGIBILITÉ

La prestation doit être chère

Le montant minimum des quotes-parts personnelles n'est pas officiellement réglementé, sauf dans le cas du remboursement des frais pour les enfants atteints de maladies chroniques. Concrètement, le FSS applique ses propres critères. Le statut socio-économique du patient n'est pas pris en compte dans la définition d'une « prestation chère ».

La prestation doit présenter une valeur scientifique reconnue et doit être prescrite par un médecin spécialiste de renom dans le domaine

A l'heure actuelle, le niveau de valeur scientifique requise varie selon que les frais médicaux sont liés ou non à une maladie rare ou à une indication rare. Pour les indications rares, la prestation doit présenter une valeur et une efficacité scientifiques reconnues par les autorités médicales et doit avoir dépassé le stade expérimental. En revanche, cette exigence n'est pas légalement établie pour les maladies rares. De l'avis des membres du Collège, la maladie est considérée comme rare lorsque sa prévalence est égale ou inférieure à 1/2000, soit le même critère que celui utilisé pour les médicaments orphelins. Cela étant, ce critère n'est pas mentionné dans la loi.

L'affection doit porter atteinte aux fonctions vitales du patient

Un élément pose question : le concept «vital » se réfère-t-il uniquement à une fonction qui est essentielle à la vie ou également au fonctionnement normal, ce qui permet de prendre en considération les aspects psycho-sociaux des maladies, de même que la qualité de vie ? Sur la base de l'échantillon de données administratives, il n'a pas été possible d'évaluer l'interprétation de cette notion.

CANAUX D'INFORMATION RELATIFS À L'EXISTENCE DU FSS

La plupart des parties prenantes interrogées ont le sentiment que l'existence du FSS est peu connue par les personnes (potentiellement) concernées. Les parties prenantes interrogées sont informées de l'existence du FSS via différents canaux. En 2007, le FSS a envoyé des brochures d'information aux assurés sociaux, aux hôpitaux, aux spécialistes, aux pharmaciens et aux associations de patients. Toutefois, nous avons constaté que la brochure du FSS ou des initiatives d'information entreprises par le FSS lui-même étaient rarement mentionnées, par les parties prenantes interrogées, en tant que canaux d'information.

COMMENT LES PARTIES PRENANTES ÉVALUENT-ELLES LA PROCÉDURE DE DEMANDE ET LE PROCESSUS DE DÉCISION ?

Clarté des critères d'éligibilité

Les répondants s'accordent à dire que les critères légaux et les exigences d'éligibilité sont susceptibles de différentes interprétations.

Durée

Il s'est révélé impossible d'évaluer la durée totale du traitement d'une demande car les données disponibles concernent uniquement l'évaluation effectuée au niveau-même du SFF. Cela étant, la durée totale de la procédure est jugée problématique par de nombreux répondants. Le goulet d'étranglement est perçu au niveau des mutualités plutôt que du FSS. Les résultats de l'analyse des données confirment partiellement cette impression puisqu'ils révèlent qu'entre 2004 et 2008, quelque 90% des cas étaient traités dans le mois par le SFF. Il convient néanmoins de souligner qu'aucune donnée n'est disponible quant à la raison d'être du probable goulet d'étranglement dans la procédure du FSS. Le fait que le même contrôle soit effectué tant au niveau des mutuelles qu'au niveau de l'institution d'assurance est un facteur retardant.

Les autres facteurs explicatifs sous-jacents pourraient être les suivants: le dépôt tardif auprès de la mutuelle, par le patient, des documents requis; le retard au niveau du service financier de l'hôpital qui doit produire la facture…

Autre question problématique évoquée par les parties prenantes: l'absence de procédure rapide pour les personnes ayant besoin d'urgence d'un traitement médical, d'un dispositif médical ou d'un médicament particulier. De surcroît, les demandes de renouvellement doivent suivre la même procédure administrative que les nouvelles demandes.

Charge administrative

La charge administrative est rapportée comme se situant essentiellement au niveau des services sociaux des hôpitaux et est considérée comme inutilement lourde par les répondants. Au niveau du patient, l'exigence d'une « déclaration sur l'honneur », attestant qu'il ne bénéficie pas d'autres sources de remboursement, risque de poser problème. En effet, elle peut entraîner un non-remboursement si le patient est décédé dans l'intervalle.

Rapportage et transparence

Les décisions négatives d'un des membres du Collège (le plus souvent le médecin dirigeant) sont contresignées par un autre membre du Collège. Une telle procédure est considérée comme suffisante par les membres du Collège, mais ils déclarent que le rapportage et le suivi de la motivation de ces décisions pourraient être plus systématiques. Les décisions déléguées aux mutualités locales ne sont pas rapportées de manière systématique au FSS. En conséquence, il n'existe pas de contrôle sur l'application uniforme de ces décisions.

Un autre problème qui entrave la transparence est l'absence d'obligation légale de rendre public le rapport annuel du FSS.

Implication des parties prenantes

Le FSS est perçu comme une entité très distante, voire carrément totalement absente du paysage habituel des soins de santé. Une représentation physique des patients au niveau du FSS par leur médecin traitant spécialiste est impossible. En outre, les personnes interrogées indiquent qu'il n'existe pas de contacts avec les groupements de patients, la profession médicale ou les services sociaux hospitaliers. Cependant, il est en principe possible de prendre contact avec le FSS; son numéro de téléphone et son adresse e-mail sont renseignés sur le site Internet de l'INAMI.

Il n'y a pas de notification de la décision au service social ou au médecin traitant qui ont – concrètement – introduit la demande au nom du patient. Une telle information est pourtant considérée comme indispensable par ces parties prenantes.

Expertise au niveau du FSS

Les médecins interrogés se demandent notamment si l'expertise du Collège des médecins directeurs est suffisante. La diversité et la rareté des maladies/indications rendent extrêmement difficile l'évaluation par un même panel « d'experts ». Même si la possibilité pour le Collège du FSS de consulter des experts externes existe, elle semble rarement utilisée. Toutefois, l'avis de la CRM est sollicité.

Par ailleurs, le médecin dirigeant du FSS estime que la qualité des prescriptions, des rapports médicaux et des preuves avancées est souvent insuffisante, ce qui complique la juste évaluation du dossier par les membres du Collège.

ETUDE DE FILETS DE SECURITE (COMPARABLES) DANS DIFFÉRENTS PAYS ETRANGERS

Nous n'avons trouvé aucun système comparable au FSS en France, aux Pays-Bas ou en Espagne. Une comparaison des modalités de remboursement d'un échantillon des produits remboursés par le FSS nous apprend que, dans les pays étudiés, certains produits ne sont pas remboursés. Il existe toutefois dans ces pays des mécanismes particuliers ayant pour vocation d'assurer un accès précoce aux nouveaux médicaments et la prise en charge des maladies rares.

SYSTÈME D'ACCÈS PRECOCE AUX NOUVEAUX MÉDICAMENTS

En France, les autorisations temporaires (ATU) permettent l'utilisation de médicaments, en dehors du cadre des essais cliniques, sans autorisation de mise sur le marché français (AMM), que ces médicaments possèdent ou pas une AMM à l'étranger. Seuls les médicaments qui sont prescrits pour le traitement de maladies graves ou rares, auxquels il n'y a pas d'alternative et pour lesquels une étude de coût-efficacité a donné des résultats positifs, sont pris en considération pour les ATU. L'utilisation non indiquée sur la notice de médicaments n'entre pas en ligne de compte pour le remboursement. L'ATU peut être délivrée dans un délai très court par l'Agence française de sécurité sanitaire des produits de santé (AFSSAPS). Elle peut être nominative pour un patient (à la demande du médecin prescripteur) ou concerner une cohorte homogène de patients à la demande d'une société pharmaceutique. Une ATU de cohorte doit être assortie d'une demande concomitante d'AMM ou de l'intention de soumettre un dossier de demande d'AMM dans un avenir proche. De plus, une ATU de cohorte est subordonnée à la mise en œuvre d'un protocole pour l'usage thérapeutique et la collecte d'informations.

Les médicaments ATU peuvent uniquement être délivrés par les pharmacies hospitalières, aussi bien aux patients hospitalisés qu'ambulatoires. Le financement des médicaments utilisés à l'hôpital est intégré dans le budget des hôpitaux à travers la dotation financement des missions d'intérêt général et d'aide à la contractualisation. Cette dotation peut être augmentée pour couvrir des dépenses exceptionnelles et imprévues associées à l'achat de médicaments ATU.

Les médicaments ATU pour les patients ambulatoires sont remboursés à 100% par l'assurance soins de santé sur la base du prix d'achat (prix conventionné entre l'hôpital et le laboratoire pharmaceutique), majoré d'une marge forfaitaire pour les frais administratifs et de distribution par l'hôpital, la TVA venant en sus du total.

En Belgique, un accès précoce aux médicaments est possible via le FSS dans des cas individuels. Pour des groupes de patients, un tel accès précoce n'est possible que dans le cadre de l'usage compassionnel (pour les médicaments sans AMM en Belgique) ou de programmes médicaux d'urgence (pour les médicaments possédant une AMM en Belgique pour une indication donnée, mais qui sont utilisés pour une autre indication). Ces programmes sont mis en place et financés par l'entreprise pharmaceutique, et les médicaments sont fournis gratuitement. Le FSS intervient souvent dans le remboursement de médicaments qui en réalité devraient être pris en charge par ces programmes.

PRISE EN CHARGE DES MALADIES RARES

En France, tout comme en Espagne et aux Pays-Bas, la prise en charge des maladies rares est regroupée dans des centres de références (ou certains hôpitaux spécifiques). Les dépenses pour les traitements et les médicaments sont inclues dans le budget des centres de référence.

PISTES D'OPTIMALISATION

Des débats sont en cours et portent au premier chef sur la structure et le fonctionnement actuels du FSS. L'objectif de ces discussions est d'envisager des solutions de rechange et des options éventuelles visant à optimiser la gestion des soins très onéreux, médicalement indispensables, non remboursés par l'assurance obligatoire.

La procédure du FSS exige que l'initiative vienne du patient ou de son médecin. Une telle approche présente un énorme inconvénient, à savoir que tous les patients potentiellement éligibles pour un remboursement ne seront pas touchés. En revanche, le remboursement par l'assurance obligatoire est automatique pour tous les services et toutes les prestations couverts par la nomenclature.

RÉVISION DES CATEGORIES

Remboursement des frais médicaux supplémentaires pour les enfants atteints de maladies chroniques: retour à l'assurance obligatoire ?

Bien qu'il existe des arguments solides pour considérer les "enfants" comme une catégorie à prendre en compte de manière spécifique, on peut se poser la question de savoir si une distinction par âge n'entraîne pas une discrimination. Cette distinction entraîne en outre que le remboursement des frais puisse être brutalement interrompu une fois que les enfants ont atteint l'âge adulte (> 19 ans). En tout état de cause, dès que l'enfant a atteint l'âge adulte, une quote-part individuelle annuelle maximale est portée en compte. Les frais découlant de l'Epidermolysis Bullosa sont toutefois intégralement remboursés, quel que soit l'âge du patient.

Remboursement des frais médicaux pour des soins à l'étranger: suppression de la discrimination?

Dans son rapport annuel pour 2007, le FSS indique que les frais remboursés dans cette catégorie couvrent essentiellement le coût des déplacements vers les pays voisins. Une telle situation pourrait créer une discrimination par rapport aux patients soignés en Belgique dont les frais d'hébergement et de déplacement ne sont pas remboursés et ce malgré le fait que dans certains cas, la distance entre leur domicile et l'hôpital peut se rapprocher de, voire dépasser, la distance jusqu'à un hôpital situé dans un pays voisin.

Implants médicaux novateurs et techniques novatrices : interaction avec la catégorie 5 de l'art. 35 § 3 de la nomenclature?

Pour cette catégorie, la procédure du FSS présente certains points communs avec la catégorie 5 de l'art. 35 § 3 de la nomenclature, qui prévoit un remboursement conditionnel pour les implants et les dispositifs prometteurs dont l'efficacité et la sécurité cliniques n'ont pas encore été établies. Les principales différences par rapport à la catégorie 5 est que la procédure du FSS prévoit une période de remboursement limitée de deux années et que le remboursement se fait au cas par cas.

Maladie ou indication rare: prendre plutôt en considération les soins médicaux onéreux consécutifs à un traitement médical justifié ?

La catégorie 'maladie rare ou indication rare' bride le champ d'action du FSS. A part des considérations de contraintes budgétaires, aucune raison objective ne justifie la non couverture de cette seule catégorie par l'assurance soins de santé obligatoire. Si cette catégorie devait être élargie aux soins médicaux coûteux résultant de traitements justifiés, il faudrait être attentif aux usages abusifs qui pourraient en être faits. S'il n'y a pas d'AMM, et/ou pas de remboursement du traitement, du médicament ou de l'implant, par l'assurance obligatoire pour une affection ou une indication fréquentes, ce sera souvent parce que il y a trop peu de preuves de leur efficacité ou parce que les firmes elles mêmes n'y croient pas. Il faut alors éviter que les patients soient exposés à de telles technologies.

UN SYSTÈME DE FRANCHISE POUR LES DÉPENSES MÉDICALES ELEVÉES CONSECUTIVES À UN TRAITEMENT MEDICAL JUSTIFIE

Il est manifeste que le FSS est un système résiduel qui a pour but d'éviter que des personnes encourent des dépenses catastrophiques associées à des soins médicaux justifiés. Faut-il moduler le niveau de dépenses au-delà duquel ces dernières sont considérées comme catastrophiques en fonction des revenus du patient ou du ménage? La question reste discutable.

Il semble n'exister aucune raison objective pour laquelle le FSS devrait se limiter aux personnes souffrant d'une maladie ou d'une indication rare, par exemple. Sur cette base, on pourrait envisager d'étendre le champ d'application aux traitements ou affections médicales justifiées. Pour préserver la viabilité financière du système, on pourrait opter un risque propre (franchise). Au-delà de ce seuil, le remboursement serait intégral. Pour éviter qu'un patient s'appauvrisse en raison d'une affection chronique, une diminution de la franchise déductible pourrait être envisagée dans le cas où des dépenses élevées sont encore encourues durant deux années consécutives ou plus.

Outre les limitations inhérentes aux critères d'éligibilité, les dépenses suivantes pourraient rester exclues du système :

- les suppléments actuellement exclus du système du FSS.
- les coûts déjà supportés par d'autres assurances ou systèmes de remboursement.

Une évaluation correcte des critères d'éligibilité restant indispensable, le passage à un système de franchise n'implique pas que la procédure actuelle de gestion au cas par cas disparaîtra ou subira un changement radical. Il convient de prévoir des mesures transitoires pour les patients transférés d'un système à l'autre.

UNE POLITIQUE COHÉRENTE DE REMBOURSEMENT DES MÉDICAMENTS

Actuellement, les médicaments sont souvent remboursés par le FSS pendant des années, sans exigence de recherches scientifiques ultérieures ou sans discussion sur le prix. Les sociétés pharmaceutiques ne sont pas toujours pressées de déposer une demande de remboursement auprès de l'assurance obligatoire, à savoir auprès de la CRM. En effet, l'introduction d'une telle demande est onéreuse et l'efficacité réelle est difficile à démontrer puisque seul un petit nombre de patients est concerné. Une demande de remboursement (ou d'élargissement de ses indications) pour un médicament dans le système d'assurance obligatoire comprend également une discussion sur le prix. Le fait que des produits pharmaceutiques soient remboursés par le FSS pendant une période très longue peut avoir un effet collatéral sur le prix d'un médicament qui n'a jamais été négocié. Avec un risque d'injustices entre les patients souffrant d'une maladie ou présentant une indication pour lesquelles ils doivent verser une quote-part personnelle, alors que les patients atteints d'une maladie rare ou d'une indication rare bénéficieront dans un large mesure d'un remboursement par le FSS.

Dans ce contexte, les producteurs utilisent parfois aussi le FSS de manière impropre, en cas de rejet du prix proposé pour le médicament ou matériel médical en question. Le circuit normal étant délibérément contourné, le patient n'a d'autre recours que d'introduire une demande de remboursement auprès du FSS. De telles situations plaident en faveur d'une plus grande cohérence dans la politique du médicament et d'une collaboration systématique entre les instances actives en matière de remboursement et de mise sur le marché des médicaments ou des implants, comme par exemple la CRM, le Collège des médecins pour les médicaments orphelins et l'Agence Fédérale des Médicaments et des Produits de Santé.

RECOMMANDATIONS DU KCE[a]

Le fonctionnement actuel du FSS pourrait être amélioré de la manière suivante :

- **Définition de critères d'éligibilité plus clairs**
  - **Les montants minimum, tant par épisode que par an, qui sont considérés comme « onéreux » au niveau du FSS devraient être rendus explicites.**
  - **L'exigence selon laquelle un traitement remboursé doit posséder une valeur scientifique reconnue devrait être décrite davantage dans les détails et s'appliquer de manière cohérente. Dans le même ordre d'idées, la notion de 'stade expérimental' mériterait d'être précisée. Enfin, la mesure selon laquelle un traitement doit être 'vital', requis ou indispensable pour être éligible aux fins d'un remboursement par le FSS devrait également être précisée.**
  - **La notion de 'rare' devrait aussi être peaufinée.**

Optimisation de l'expertise par la collaboration

- **L'expertise disponible au niveau du FSS devrait être optimisée afin de permettre une prise de décision adéquate. Une option pourrait être de créer des panels ad hoc d'experts (nationaux ou internationaux) dans les domaines spécifiques relevant du FSS. Une autre possibilité consisterait à instaurer une collaboration structurelle avec les différentes instances qui interviennent dans le remboursement et la mise sur le marché des médicaments et implants, telles que la CRM, le CMDOD, Agence Fédérale des Médicaments et des Produits de la Santé (AFMPS), etc. Cette mesure aurait pour effet non seulement d'optimiser l'utilisation de l'expertise disponible, mais aussi de réduire le risque de décisions contradictoires.**
- **Une implication du médecin prescripteur, lui offrant la possibilité de formuler des commentaires et d'argumenter son avis médical, pourrait être envisagée. Une telle mesure permettrait aux membres du Collège d'étoffer leur connaissance de l'indication ou maladie rare en question. L'introduction de telles procédures devrait toutefois relever le défi spécifique de ne pas créer de charge administrative supplémentaire et de ne pas prolonger le délai de prise de décision.**

- **Raccourcissement des procédures et allègement de la charge administrative**
  - **Des doublons aux différents niveaux intéressés devraient être évités. Un point de contact central où toutes les données sont centralisées serait préférable. La soumission des demandes devrait se faire totalement par voie électronique, y compris des signatures électroniques. Il faudrait encourager les services sociaux des hôpitaux et des mutualités à faciliter l'introduction des demandes pour les patients ou leurs membres respectifs.**

a Le KCE reste seul responsable des recommandations faites aux autorités publiques

- **Dans le droit fil des constats posés par le KCE dans son rapport sur les médicaments orphelins, toutes les demandes de remboursement par le FSS pourraient être introduites auprès d'un point de contact central unique au sein de l'INAMI. Ce point de contact pourrait être le même pour les demandes de remboursement par le FSS et les demandes pour les médicaments orphelins, l'objectif étant d'aligner les décisions de remboursement sur les médicaments orphelins. Ce point de contact pourrait faire office de centre de coordination et référer les médecins ou les patients atteints d'une maladie rare vers le médecin expert ou le centre de référence idoines. Idéalement, cette nouvelle structure devrait garantir une application cohérente des critères de remboursement.**
- **Alors qu'à l'heure actuelle, les renouvellements suivent les mêmes procédures que les demandes, leur parcours pourrait être considérablement raccourci dans le cas où de nouvelles preuves n'existent pas ou ne sont pas nécessaires.**
- **Pour les personnes ayant un besoin urgent d'un traitement médical, d'un dispositif ou d'un médicament particulier, on pourrait envisager une procédure accélérée.**

Meilleure transparence

- **Les rapports annuels contenant les données anonymes et consolidées devraient être publiés dans le but d'accroître la transparence pour toutes les parties intéressées. Un site Internet central reprenant des informations utiles et des liens vers les centres de référence et les organisations de patients pour chaque maladie rare serait un outil très précieux. D'autre part, les services sociaux des mutualités et des hôpitaux devraient jouer un rôle plus important dans l'information du patient au sujet du FSS, car ces services sont souvent le premier interlocuteur des patients qui sont confrontés à des dépenses exceptionnelles et considérables.**
- **Une remontée d'informations directe sur la décision (si nécessaire, avec l'accord du patient) jusqu'au médecin traitant spécialiste et/ou le service social augmenterait l'engagement des parties respectives et pourrait mener à un recours plus systématique au FSS.**

Les questions suivantes méritent par ailleurs réflexion pour l'avenir :

- **Un système d'accès précoce à de nouveaux médicaments pour une indication particulière, assorti de conditions et similaire à celui appliqué en France, ne pourrait-il pas être envisagé ? Ne conviendrait-il pas de coordonner les autorisations nominatives et de cohorte au sein d'une seule entité ?**
- **Ne faudrait-il pas veiller davantage à éviter toute discrimination dans les critères d'éligibilité (rareté de la maladie, âge du patient, indication) ?**
- **Les demandes de remboursement pour des dispositifs médicaux innovants ne devraient-elles pas suivre la procédure de l'art. 35 § 3 de la catégorie 5, dans les cas où celle-ci se justifie et est applicable ?**
- **Dans le cas des médicaments pour lesquels le remboursement par le FSS fait l'objet de demandes fréquentes, et dans le but de prévenir un usage impropre du FSS en qualité de salle d'attente et/ou de contournement du système régulier, ne faudrait-il pas exiger de la firme pharmaceutique présenter une demande d'AMM (si ce n'est pas encore le cas) et de s'engager à introduire une demande auprès de la CRM ?**
- **La mission du FSS en tant que filet de sécurité ultime contre des dépenses catastrophiques ne devrait-elle pas être affermie et rendue plus cohérente :**

- **en le rendant applicable à tous les traitements médicaux très onéreux en dehors de l'assurance obligatoire, à condition qu'ils soient considérés comme efficaces et justifiés ? Cependant, on devrait alors veiller à ce que des produits ou traitements inefficaces ou insuffisamment évalués ne soient pas remboursés (et mis à disposition des patients) pour des affections ou des indications fréquentes.**
- **en transformant le mécanisme de remboursement en 'système de franchise' doté d'une disposition supplémentaire (par exemple, une fraction déductible inférieure) pour les dépenses élevées chroniques ?**

- **Ne conviendrait-il pas de créer des centres de référence chargés de structurer le diagnostic et la prise en charge des patients souffrant d'une maladie particulièrement rare ? Les soins dispensés dans ces centres (comprenant le traitement mais aussi l'usage de médicaments non inclus dans la nomenclature) pourraient faire l'objet de et être financés via une convention avec l'INAMI. Le champ d'action du FSS pourrait dans ce cas être restreint aux maladies rares pour lesquelles aucun centre de référence n'a été conventionné par l'INAMI.**

Scientific summary

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LIST OF ABBREVIATIONS

AEMPS	Agencia Espanola de Medicamentos y Productos Sanitario
AFSSAPS	Agence Française de Sécurité Sanitaire des Produits de Santé
AIDS	Acquired Immuno Deficiency Syndrome
ALD	Affectation de longue durée
AME	L'aide Medicale d'Etat
ATU	Authorisation for temporary usage
ASMR	L' amélioration du Service Médical Rendu/improvement in clinical value
AWBZ	Algemene wet bijzondere ziektekosten
BBC	Special Assistance Committee
BOKS	Belgian patient organisation for children and adults with metabolic disorders
CANAM	La caisse nationale d'assurance maladie des professions indépendants
CE	European Conformity
CEPS	Comité economique des Produits de Santé/French Health Care Products Economic Committee
CIP	Club Inter Pharmaceutique
CMU	Couverture maladie universelle
CPA	Dutch Committee for Pharmaceutical Aid
CSUR	Centros Servicios o Unidades de Referencia (Spanish reference centers)
CTZ	College Toezicht Zorgverzekeringen/Dutch supervisory board for Health insurance
CVZ	College van zorgverzekeraars/College of Health insurers
DEBRA	Dystrophic Epidermolysis Bullosa Research Organisation
DRG	Diseases Related Groups
DRC	Drug Reimbursement Commission
DTC	Diagnosis and treatment Combinations
EB	Epidermolysis Bullosa

EMEA	European Medicine Agency
EU	European Union
EURORDIS	European Organisation for Rare Diseases
FEDER	Federation Espanola de enfermedades rares/Spanish federation for rare diseases
GIS	French institute for rare diseases
GP	General Practitioner
GVS	Geneesmiddelenvergoedingssysteem/Dutch medicines reimbursement system
HAS	Haute Autorité de santé/French High Health Authority
HCIB	Dutch Health Care Insurance Board (College voor zorgverzekeringen)
HKC	Hoge kosten compensatie/High Cost Compensation
HKV	Hoge kosten verevening
ICD9-CM	International Classification of Diseases, 9th revision, Clinical Modification
IIER	Spanish research institute for rare diseases
INSALUD	National Health System Structure/Central national health institute
LUSS	Walloon patient platform
MAB	Maximum billing/invoice
MSA	Mualité Sociale Agricole
NEMA	Flemish patient organisation for neuromuscular diseases
NHS	National Health Service
NIHDI	Belgian National Institute for Health and Disability Insurance
	(Rijks Instituut voor Ziekte en Invaliditeitsverzekering/Institut National d'Assurance Maladie et d'Invalidité)
NMRC	Neuromuscular Reference Centers
NVZ	Nederlandse vereniging van ziekenhuizen/Dutch organisation of hopsitals
NYHA	New York Heart Association
OCMW/CPAS	Public centers for social welfare in Belgium
ORPHANET	The portal for rare diseases and orphan drugs

PH	Pulmonary Hypertension
RADIORG.BE	Rare Diseases Organisation Belgium
SMR	Service Medical Rendu
SF	Sickness Fund
SSF	Belgian Special Solidarity Fund (Bijzonder Solidariteits fonds/Fond Spécial de Solidarité)
UCD codes	Communes de dispensation utulisées à l'hôpital
VAPH	Flemish Agency for Persons with Disabilities
VIH	Le virus de L'immuno déficience humaine
VPP	Flemish Patient Platform

1 INTRODUCTION

The Special Solidarity Fund (SSF) was established by law as part of the National Institute for Health and Disability Insurance (NIHDI) and is operational since 1990. The Fund complements the compulsory health insurance coverage and serves as a social care net covering high cost rare diseases excluded from the universal insurance system. One can make an appeal to the SSF if all other possible sources of reimbursement have been exhausted. Additionally, several criteria have to be met in order to be eligible for reimbursement. Reimbursement can be granted for certain costs related to rare diseases, rare indications or the application of innovative techniques, which are not (yet) covered by the compulsory health insurance system in Belgium or any other channel (private insurance – reimbursement abroad). The target population of the SSF are seriously ill patients for whom an expensive but not (yet) reimbursed treatment is essential. Chronically ill children (children below 19 years suffering from cancer, renal insufficiency or any other life threatening disease, requiring a continuous or repetitive treatment of at least 6 months) are a specific target group of the SSF. In this case the SSF can reimburse additional costs as soon as €650 out-of-pocket payments have been paid on a yearly basis.

The current procedure requires patients – often through the treating physician or the social service of the hospital where the treating physician works – to submit an application to the advising physician of their local sickness fund. After its submission, the application passes through the national sickness fund and is finally transferred to the SSF. The "College of Medical Directors" is the decision-making body within the SSF. This body assesses the individual application files and takes the final decision regarding reimbursement. Then the SSF informs the local sickness fund and in case of a positive decision, the patient is reimbursed within 15 days after the decision.

The Minister of Social Affairs determines the budget of the SSF on a yearly basis.

2 DESCRIPTION OF THE SSF

2.1 THE SSF IN THE BELGIAN HEALTH CARE SYSTEM[1]

2.1.1 General overview of the Belgian Health Care system

The Belgian health care system is mainly organized at two levels, i.e. federal and regional. Responsibility for health care policy is shared between the federal Government, exercised by the Federal Public Service Health, Food Chain Safety and Environment (former Ministry), the Federal Public Service Social Security, the National Institute for Health and Disability Insurance (NIHDI), and the Dutch-, French- and German-speaking community Ministries of Health.

The main responsibilities for social security, compulsory health insurance, pharmaceutical policy and hospital legislation are concentrated at the federal level. This is certainly the case for the Special Solidarity Fund (SSF) as the federal Government is responsible for the regulation and financing of the compulsory health insurance, registration of pharmaceuticals and their price control.

The Belgian health system is primarily funded through social security contributions and taxation and is based on the principles of equal access and freedom of choice, with a Bismarckian-type of compulsory national health insurance, which covers the whole population and has a very broad benefits package. Compulsory health insurance is combined with a private system of health care delivery, based on independent medical practice, free choice of service provider and predominantly fee-for-service payment.

By means of the Sickness Funds Act, sickness funds are entrusted with a central position in compulsory health insurance. They have to control the conformity of health care expenditure with the legal regulations.

Private-for-profit health insurance companies account for only a small part of the (mainly complementary) health insurance market. In the field of voluntary health insurance, the sickness funds compete with commercial insurance companies.

2.1.2 The SSF as part of the National Institute for Health and Disability Insurance

The National Institute for Sickness and Disability Insurance is a public body accountable to the Minister of Social Affairs and Public Health. This institute is responsible for the general organization and financial management of the compulsory health insurance. The NIHDI determines the rates and services which are reimbursed in the national established fee schedule (nomenclature), with final ratification by the Minister of Social Affairs in a Royal Decree.

Of the many organs, connected to the medical care service (Health Care department), the 3 following structures play an important role: the Commission for budget control, the Scientific Council and the College of Medical Directors.

This College of Medical Directors has different tasks to perform, mainly related to the rehabilitation sector, the sector retraining and the SSF.

The College of Medical Directors administers the Special Solidarity Fund whose scope was substantially amended in 2005 according to the care needs regarding the care provided in Belgium. As of 2005 the scope of SSF interventions are rare indications, rare diseases, with specific application rules if these require continuous and complex care, innovative technologies and the additional costs for the medical treatment of very seriously chronically ill children.

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[1] This chapter is mainly based on the report Corens D. Health system review: Belgium. Health Systems in Transition, 2007[1] and information available on the NIHDI website (http://www.riziv.fgov.be; http://www.inami.fgov.be)

2.1.3 Position of the SSF in the Belgian Health System

2.1.3.1 The SSF position in practice: unique but complex

Although the SSF is fully embedded in the NIHDI, the SSF complements the Belgian compulsory health insurance system and reimburses certain medical expenses for rare diseases, rare indications and innovative techniques which are not (yet) refunded by the compulsory health insurance. This situation creates a strange and unique positioning of the SSF within the national health system. The SSF belongs structurally to the compulsory health care insurance system (NIHDI) but performs activities (reimbursement) which fall outside the regular system.

Secondly, the SSF only intervenes when all other options/channels for reimbursement have been exhausted including reimbursement from private health insurance companies. This condition emphasizes that the scope of the SSF is outside the compulsory health insurance system but it is on the other hand confusing as the SSF belongs to the compulsory health insurance structure. The SSF is the last additional safety net to "ordinary" insurance coverage for medical care.

This unique position makes the SSF heavily dependent on the functioning of the various councils, committees or colleges and the evolution of the benefit package as a whole within the regular health insurance system. The services that are covered by the compulsory health insurance are described in the nationally established fee schedule (the "nomenclature"), which is extremely detailed and lists more than 8000 procedures and types of products. For each service, the identification number, contractual fee and reimbursement rate are specified. Services not included in the fee schedule are not reimbursable. At regular intervals, new treatments or products are introduced into the benefits package and treatments that have become obsolete are removed. When the fee schedule/nomenclature is adapted too slowly to evolutions in the general medical practice, this will affect the SSF by keeping the reimbursement of these services and medical acts in the scope of action of the SSF instead of inclusion into the regular reimbursement system (compulsory health insurance).

On the other hand decisions taken by the SSF can also influence negotiations and activities of the committees, councils and colleges of the compulsory health insurance system. This could be the case for the negotiations with the pharmaceutical companies on the reimbursement level for new drugs led by the Drug Reimbursement Commission (DRC/CTG/CRM). In order not to influence these negotiations, the SSF, if it decides to reimburse a drug before acceptance in the compulsory health insurance, can decide to accept only a part (mostly a percentage) of the cost that is charged by the pharmaceutical company.

One can notice that the relation between the SSF and other compulsory health insurance committees is bidirectional.

2.1.3.2 Other protection mechanisms and the relation to the SSF

Regulation on orphan drugs

For orphan drugs the Belgian compulsory health insurance system introduced a specific reimbursement framework. The "College of medical directors on orphan drugs" (CMDOD) assesses the individual right of the patient to the reimbursement of an orphan drug, as far as the reimbursement conditions require such an assessment and as far as the advising physician of the patient's sickness fund requested the advice of the College. The action field of the College interacts with the action field of the SSF. If the CMDOD decides on the reimbursement of an "orphan drug" for a particular indication for an individual patient, the SSF will no longer decide on the reimbursement of this drug, not even for indications the CMOD did not decide on (off label use of the respective orphan drug). This implies that patients potentially benefiting from SSF reimbursement of a drug for a specific indication before the respective drug was accepted as an orphan drug, will no longer be reimbursed if the CMDOD did not include that particular indication in its decision[2].

Preferential treatment and OMNIO

Patients with 'preferential treatment' pay reduced co-payments (part of the official tariff that is not reimbursed). The reduction depends on the type of expenditure (GP, specialist, drugs, hospital…). Originally, the system of preferential treatment was restricted to patients with a specific social status (pensioners, widow(er)s, persons with disabilities and orphans), for which the gross taxable income of the family did not exceed a yearly-adapted limit. In 1997 and 1998, the benefit of the preferential tariff system was extended to other groups as long term unemployed people, persons entitled to allowances for handicapped persons or for assistance for the elderly and persons getting assistance from the Public centers of social welfare. Since 2007, the system is further extended. The newly introduced OMNIO-status guarantees preferential treatment to all households below a certain income level. Persons benefiting from the OMNIO-status will get a higher reimbursement of the medical costs within the general health insurance system[3]. The right to this OMNIO-status solely depends on the family income level. Specifically, if the gross taxable family income is lower than €14 339.94 (for 2008) the OMNIO-status is applicable. The maximum income level is majored with €2 654.70 per family member that is not the applicant.

As such there is no interaction between the SSF reimbursements and the OMNIO-status since costs reimbursed by the SSF are no part of the general (compulsory) health insurance system. Reimbursement of costs by the SSF is completely indifferent from the family income level. The fact a patient benefits from the OMNIO-status has no effect on it.

MAB (maximum billing)

The MAB limits the maximum annual medical costs for families to a ceiling amount.[4] Once the expenditure for medical care reaches the ceiling amount on a yearly basis, the personal share (co-payment) of the costs of medical treatments is no longer applicable. The costs that are taken into account are a subset of the costs as regulated in the nationally established fee schedule (the "nomenclature"). The MAB is applicable to all patients and is part of the compulsory health insurance. As every family is entitled to the MAB, patients that benefit from the OMNIO-status, also can benefit from the advantages of the MAB if the ceiling amount is reached.[5] In order to give a supplementary protection to children who are confronted with high health care expenses, an individual MAB-right for children was installed. Irrespective of the MAB-ceiling for the de facto household they are living in, children are always entitled to individual reimbursement of their co-payments that exceed a ceiling of €650. The MAB right for children interferes with the reimbursement of extra medical costs for chronically ill children by the SSF, where a ceiling of €650 was also applied. In principle reimbursement by the SSF can only be granted if there was no other direct or indirect source of reimbursement. It is conceivable however that costs eligible for MAB application are subject of applications for reimbursement introduced to the SSF. It is up to the local sickness funds to watch the overlap between the two systems. The SSF can reimburse the co-payments that were not taken into account for the calculation of the MAB ceiling, as well as the delivery margin[2] (10% of the price) and the safety margin[3] for costly implants.

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2 For reimbursable implants, hospitals can charge a delivery margin of 10% of the sales price including VAT, with a maximum amount.

3 The safety margin is a percentage of the reimbursement tariff and equals the maximum amount of supplements to be charged by hospitals.

Compassionate use / medical urgency programs

Compassionate use of drugs concerns the treatment of patients with drugs that are not yet reimbursed or available in Belgium.[6]

Compassionate use can be applied in two cases:

- Programmes of compassionate use: making available, for compassionate reasons, of a medicinal product that can qualify for the centralized procedure to a group of patients with a chronically or seriously debilitating disease or whose disease is considered to be life-threatening, and who cannot be treated satisfactorily by an authorised medicinal product. The medicinal product concerned must either be the subject of an application for a Marketing Authorisation in accordance with Article 6 of the European Regulation laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[7] or must be undergoing clinical trials.[8]
- The Medical Need Programmes: making available a medicinal product to a group of patients with a chronically or seriously debilitating disease or whose disease is considered to be life-threatening, and who cannot be treated satisfactorily by an authorised medicinal product. The medicinal product concerned must have a Marketing Authorisation but
  - either the given indication has not been authorised yet, or
  - although authorised, the medicinal product is not yet available on the market in this indication.[8]

The essential difference between the two programmes is that Compassionate Use concerns medicinal products which do not yet have obtained a Marketing Authorisation in Belgium, unlike the Medical Need Programme, which concerns medicinal products which have a Marketing Authorisation in Belgium for a given indication. In order for a medicinal product to be considered for compassionate use, the market authorization holder will have to introduce a demand that will be reviewed and approved by one of the Belgian ethics committees. The compassionate use treatment will be prescribed by a physician: the hospital can require approval for the individual patient by the local ethics committee. In both cases the pharmaceutical companies bear the costs for the medicinal products.

Keypoints: The SSF in the Belgian health care system

- **The Belgian health care system is organized at federal and regional level. The federal level is responsible for the regulation and financing of the compulsory health insurance that is organized by the NIHDI.**
- **The SSF is a part of the NIHDI as a complement to the compulsory health insurance system.**
- **Reimbursement of orphan drugs is organized separately from the SSF but interacts. The "College of medical directors on orphan drugs" mostly decides on the individual right of the patient to the reimbursement of an orphan drug.**
- **The compulsory health insurance has systems as the OMNIO status and the MAB to protect vulnerable groups of patients with high medical costs.**

2.2 LEGAL CONTEXT OF THE SSF

The SSF was established by law and is operational since 1990 (art. 13 and 14 Law 22 December 1989).[9] Over the years regulation on the SSF has changed.[10, 11] The actual regulatory framework of the SSF is the law of 27 April 2005 (art. 2-6 [12]). The entire text of the law can be found in annex 9.1.

Since 2000 interventions that are included in the nomenclature but are not reimbursable by the compulsory health insurance because the patient does not satisfy the conditions, are eligible for reimbursement by the SSF (art. 22 Law 24 December 1999).[13])

In 2001 a specific target group was created: patients suffering from Epidermolysis Bullosa. For this patient group, the criteria rare and expensive were omitted. The same law inserted the regulation of continuous or chronic treatments and specific rules regarding the procedure, the advance and the budget were drafted.

The program law of 24 December 2002 [11] inserted the possibility to create limitative lists of reimbursable interventions and the possibility to set a maximum amount of reimbursement. Moreover specific rules for chronically ill children were created and the term of introduction of the reimbursement request was limited to 3 year after the month in which the medical interventions had taken place.

In 2005, the field of application of the SSF was extended with new regulations. The different costs eligible for reimbursement are elaborated in the next section. Apart from article 25quinquies that applies for interventions as from 1 January 2004, the 2005 legislation applies to interventions as from 1 April 2005.

Next to the specific legal framework setting different categories of medical costs eligible for reimbursement and the specific reimbursement criteria, decisions by the SSF are also framed by other legislation, particularly in the domain of drugs (e.g. regulation on orphan drugs; drugs registered in Belgium, but not available in Belgium can not be imported,. ..).

2.3 CATEGORIES OF MEDICAL COSTS REIMBURSED BY THE SSF

The cases in which medical costs are eligible to reimbursement by the SSF are stipulated in the law. The situation in which reimbursement can be granted as well as the criteria the treatment has to meet, are very specific. Some costs, such as honorarium supplements[4], price supplements (e.g. supplements to medical material) and room supplements[5], that can legally be charged to patients (according to art. 90 Hospital Law), co-payments[6] and comfort costs[7] are specifically excluded from reimbursement (art. 25, 4th section).[12] The co-payments that were not taken into account for the calculation of the MAB ceiling, as well as the delivery margin and the safety margin for costly implants however are eligible for reimbursement to chronically ill children under 19 years old (see also above).

The main categories are cost of medical treatment related to:

- rare indications (art. 25bis)
- rare diseases (art. 25ter §1) requiring a specific fysiopathological treatment
- rare diseases requiring a continuous and complex treatment (art. 25ter §2)
- innovative treatment techniques (art. 25quater)

4 i.e. amounts charged to the patients above the official honorarium tariff

5 i.e. amounts charged by the hospital for single or double rooms

6 i.e. the part of the official tariff that is not reimbursed

7 diverse non medical items for inpatients such as costs of a refrigerator, telephone or television in the hospital room

- chronically ill children (art. 25quinquies)
- medical treatment abroad (art. 25sexies)

Below we give an overview of the criteria, applicable for the different items.

2.3.1 Reimbursement of treatment costs for rare indications (art. 25bis)

Reimbursement of the treatment costs for rare indications has to meet the following additional requirements:

- The treatment is expensive;
- Medical treatment is prescribed by a medical doctor specialized in the treatment of the related disorder and authorized to practice medicine in Belgium;
- Medical treatment has a scientific value and effectiveness which is largely recognized by the medical profession. The medical treatment has to have outgrown the experimental phase;
- The compulsory health insurance system can't provide an alternative;
- Medical treatment is used for an indication threatening vital functions of the patient.

2.3.2 Reimbursement of treatments costs for rare diseases (art. 25ter § 1)

The SSF can provide reimbursement of medical costs for patients suffering from rare diseases.

Additional requirements are:

- The medical treatment is considered as expensive;
- The compulsory health insurance system does not provide a therapeutic alternative treatment;
- Medical treatment is used for a rare disease that threatens the vital functions of the patient;
- The medical treatment is prescribed by a medical doctor specialized in the treatment of the specific disease and authorized to practice medicine in Belgium;
- The medical profession recognizes the treatment as the specific approach for the rare disease.

2.3.3 Reimbursement of costs for rare diseases requiring a continuous and complex treatment (art. 25ter §2)

The costs for rare diseases requiring a continuous and complex treatment can be reimbursed by the SSF.

Additional requirements for reimbursements are:

- Treatment as a whole is expensive;
- Treatment is related to a threat of the vital functions of a patient. The threat of the vital functions is directly and specifically a consequence of the rare disease;
- The compulsory health insurance system does not provide a therapeutic alternative;
- The complex treatment is prescribed by a medical doctor, specialized in the treatment of the specific disease and authorized to practice medicine in Belgium.

2.3.4 Reimbursement of expenses for innovative treatment techniques (art. 25quarter)

The reimbursement for innovative treatment techniques and medical devices does not cover expenses related to drugs.

Reimbursement is only allowed when the treatment techniques and medical devices are expensive and rare. Moreover authoritative medical bodies need to recommend the treatment and medical device as the appropriate way to treat the disease that threatens vital functions of the patient.

Additional requirements for reimbursement are:

- The medical treatment has outgrown the experimental phase;
- Based on a cost benefit-analysis, it should be clear that the medical treatment has an important and proven added value;
- The medical treatment is prescribed by a medical doctor, specialized in the rare disease and authorized to practice medicine in Belgium;
- An application to assess the medical "added value" of the treatment and/or the request for inclusion for reimbursement within the compulsory insurance system has to be submitted to the authorized Technical Council, part of the compulsory insurance system;
- The medical treatment or medical devices need to be on a limitative list approved by the Insurance Committee of the NIHDI for which reimbursement can be granted for a period of maximum one year. This maximum period of one year can be renewed once if motivated by the Insurance Committee.

2.3.5 Reimbursement of extra medical costs for chronically ill children (art. 25quinquies)

Chronically ill children are children suffering from cancer, renal insufficiency (treated via peritoneal dialysis or haemodialysis) or every other life-threatening sickness that requires a medical treatment of at least six months or a consecutive treatment of the same duration.

Other requirements are:

- Children are under 19;
- The extra medical costs of the treatment exceed €650 on an annual basis;
- The extra costs have to be related to medical treatment prescribed by a medical doctor, who treats the patient or who coordinates the treatment of the patient. This medical doctor has to be authorized to practice medicine in Belgium;
- The medical treatment has a scientifically proved added value and effectiveness that is largely recognised by authoritative medical bodies;
- The compulsory health insurance system does not provide other alternative treatments for the indication in terms of diagnosis or therapy.

2.3.6 Reimbursement of expenses for medical treatment abroad (art. 25sexies)

The SSF refunds costs for non-reimbursable medical treatment abroad, as well as travel and stay costs for the patient and for his companion, if "worthy of consideration".

Other requirements for reimbursement are:

- The advisory physician of the health insurance fund has approved the treatment of the patient abroad;
- A medical specialist, authorized to practice medicine in Belgium and specialized in the treatment of the specific disease, has prescribed the medical treatment. Prescription has to be prior to the start of the treatment.

2.3.7 Common criteria of eligibility for reimbursement

Despite the fact that the requirements are specific and cumulatively applicable within each article of the law, the following criteria show up in most categories:

- Expensive;
- Threatening the vital functions of the patient;
- Proven scientific value, effectiveness of the treatment;
- No alternative available within the compulsory health system;
- Prescription made by a medical doctor, specialized in the treatment of the related disease.

Further, the SSF does not reimburse costs related to medical treatment if the patient has any other rights for reimbursement. This actually means, the patient has to verify if he/she could benefit from any private insurance he/she subscribed or from other public insurance systems before entering a demand under the SSF regulation. The patient has to provide a declaration on honour, stating he/she does not benefit similar rights. There is however no control on the faithfulness of the declaration.

The personal financial situation of the patient is currently not taken into account, when deciding on reimbursement (or portion of reimbursement) by the SSF. As mentioned earlier financial protection measures exist within the compulsory health insurance system.

3 OBJECTIVE OF THE STUDY

The original scope of the study was to assess whether the Special Solidarity Fund (SSF) optimally reaches the target population as defined by law and to identify the unmet needs. Due to methodological reasons however, this turned out to be impossible. The overall objective of this report is to assess the operation processes of the SSF on the procedural level as well as regarding the content and to formulate suggestions for the optimisation of the operability of the SSF. Therefore this study will particularly focus on the description of its activities, the evaluation of its functioning, and possible comparable systems in a selection of foreign countries. Although the study particularly zooms in to the evaluation of the "as is" situation, it is necessary to frame it in a historic timeframe in order to isolate effects of changes in legislation and to understand the current situation. An identification and quantification of the patients that are eligible for reimbursement of their costs by the SSF but did not enter an application for whatever what reason can not be performed. A theoretical reflection of the unmet needs however falls within the scope of this study.

3.1 DESCRIPTION OF THE FUNCTIONING OF THE SSF

The first research question, related to the functioning of the SSF, has been subdivided into the following sub questions:

- What are the goals of the SSF?
- How is the budget of the SSF determined?
- How is decided whether to include certain drugs, implants and other items for reimbursement by the SSF? What is the relation with the fixed budget? How are decisions to elude items from the SSF taken?
- What is the evolution of the number of patients, applications, included items during the last 5 years?

The answers to these questions will provide a view on the internal organisation and procedures followed by the SSF and on the main stakeholders involved in the process.

Additional to this micro-economic view, the position of the SSF in the framework of our health insurance system will be taken into account. This macro-economic approach focuses more on the relation between the SSF and the compulsory health system.

3.2 EVALUATION OF THE FUNCTIONING OF THE SSF

The second research question, linked to the evaluation of the functioning of the SSF has been divided in 3 sub-questions which are stipulated below:

- What are the explicit and implicit criteria for assessment of eligibility of items and how are they interpreted?
- Is the SSF known among patients, prescribing doctors, hospital social services and the pharmaceutical industry? What are the information channels through which they get informed about the existence of the SSF?
- How is the application procedure and decision-making process evaluated by all parties concerned (e.g. clarity of criteria, duration, "customer" friendliness)?

The purpose of these questions is to make a critical assessment of the procedural and the substantive processes of the SSF based on stakeholders perception and quantitative analysis of the SSF database sample. External stakeholders include "medical specialists making use of the SSF", "hospital social services" and patient organizations playing an important role as information providers and/or application initiators toward the potential beneficiaries (patients). The pharmaceutical industry is also an external stakeholder via their link with the treating medical doctors (prescribers), the patient (provision of drugs) and the NIHDI (reimbursement of orphan drugs). The medical directors of the health insurance funds are as well internal stakeholders (members of the decision-making body of the SSF) as external stakeholders (every application of a potential beneficiary patient needs to be introduced via his/her health insurance fund to the SSF. In the methodology section the different stakeholders are described in detail.

3.3 EXPLORATION OF (COMPARABLE) SAFETY NETS IN A SELECTION OF FOREIGN COUNTRIES

The purpose of the third research question is "what lessons can be learned from a selection of foreign countries?" In order to answer this question 2 sub-questions are addressed:

- Do comparable safety nets for rare diseases exist in a selection of foreign countries and how do they function?
- How is the care of selected cases of the targeted SSF population organized in the selected countries?

4 METHODOLOGY

4.1 DESCRIPTION OF THE FUNCTIONING OF THE SSF

As indicated in the previous chapter, the description of the functioning of the SSF focuses on the organization of the SSF, its procedures and activities.

To answer these topics, we followed a qualitative and quantitative methodological approach described below.

4.1.1 Review of SSF publications

In order to get a first helicopter view on the functioning of the SSF, the existing SSF publications were reviewed. These publications were identified via the NIHDI website or trough a personal contact with the leading medical officer of the SSF. All publications were initially reviewed according to the topics included in the sub-questions (see objectives of the study). Additional relevant topics, related to the functioning of the SSF and un-clarities were listed as input for the interview with the leading medical officer of the SSF.

Documents	Source
Legislation texts (Royal Decree	Website NIHDI http://www.riziv.fgov.be/care/nl/infos/solidarity/pdf/art25.pdf
SSF brochure for the potential applicants (patients)	Website NIHDI http://www.riziv.fgov.be/care/nl/infos/solidarity/pdf/fss20060424.pdf
Annual reports from 2003 till 2007	Personal contact leading SSF officer

4.1.2 Qualitative approach by interviews and semi-structured interviews

4.1.2.1 Population

To obtain a global overview on the functioning of the SSF, we met several employees of the NIHDI/SSF that are involved in the daily activities and the procedures of the SSF.

To get more information and details on the budget cycle (drafting, approval and follow up of the SSF budget) the officer responsible for the budget of the SSF/NIHDI was interviewed.

Furthermore, we performed interviews with the leading medical officer of the SSF and the members of the College of Medical Directors, the decision-making body of the SSF to get a clear view on the decision-making process and on the criteria used within the SSF. The members of the College of Medical Directors are internal stakeholders as they take the final decision on SSF applications for reimbursement. On the other hand they are also external stakeholders as they belong to one of the 7 official health insurance funds. These health insurance funds act as intermediate between the patients (potential beneficiaries) and the SSF.

4.1.2.2 Selection of method

For the employees of the NIHDI/SSF and the leading medical officer of the SSF, individual open interviews were executed. This choice was made as the topics of the interviews were very specific and highly linked to the daily tasks and responsibilities of the interviewees. These interviews among so called "key informants" aimed to help the research team to become familiar with the SSF and to clarify the information that was already available (legislation, the brochure, the activity report). No particular analysis on the findings was carried out. This part is therefore no pure qualitative research although a qualitative methodology of data collection was used. In the same way, for the members of the College of Medical Directors, we used semi-structured interviews. The advantage of semi-structured interviews is the flexibility, allowing new questions to be brought up during the interview as a result of what the interviewee says. The set of questions are prepared (clear focus for the interviewer on the themes) but open, allowing the interviewees to express opinions through discussion.

4.1.2.3 Sampling

The sampling process for the internal SSF stakeholders was developed as follow:

- The leading medical official of the SSF: Dr. Gendreike was interviewed in order to obtain a global view on the functioning of the SSF. During this interview, 3 additional interviewees were identified and indicated to allow further and more detailed data collection.
- The following employees of the NIHDI/SSF were interviewed seen their daily involvement in specific SSF topics:
  - Ms. Van Campenhout and Mr. Noel, both responsible for the administrative preparation and follow up of SSF files
  - Mr. Vigneul, responsible for the budget follow up within the NIHDI

The selection of the members of the College of Medical Directors was based on the following logic. In Belgium there are 7 health insurance funds[8]. The five health insurance funds, representing the biggest share of the population were included in the sample. As within the socialist health insurance fund, two different persons (one Dutch-speaking and one French-speaking) are involved in the College of Medical Directors, we decided to include both of them in the interview process. The other health insurance funds have a single person handling the SSF applications in the College of Medical Directors. As a consequence we executed 6 interviews with representatives of 5 health insurance funds.

4.1.2.4 Data collection

Tool

For the employees of the NIHDI/SSF no interview guideline was developed as the purpose of the interview was to let them explain in an open way the functioning of the SSF, giving them the opportunity to go into details for their specific subtasks and/or responsibilities.

For the members of the College of Medical Directors, we used a semi-structured interview guideline, consisting of a number of orienting generic questions and a number of sub-questions in order to clarify or expand the view of the interviewee. The semi-structured interview guideline was translated in the native language of the interviewees (Dutch/French) and is included as annex 9.6.

Interview processing

The interviews were conducted by one person, in the native language of the interviewee.

Topics of the interview for the NIHDI/SSF employees

The emphasis of this open interview was to obtain a clear view on the internal organization of the SSF, the procedures followed, the criteria used, the main stakeholders involved in the process and the evolution of the activities as a result of changes in the legal context or changes in the compulsory health system.

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8 Landsbond der Christelijke Mutualiteiten/Alliance Nationale des Mutualités Chrétiennes; Landsbond van de Neutrale Ziekenfondsen/Union Nationale des Mutualités Neutres; Nationaal Verbond van Socialistische Mutualiteiten/Union Nationale des Mutualités Socialistes; Landsbond van Liberale Mutualiteiten/Union Nationale des Mutualités Libérales ; Landsbond van de Onafhankelijke Ziekenfondsen/Union Nationale des Mutualités Libres ; Hulpkas voor Ziekte- en Invaliditeitsverzekering/Caisse Auxilliaire d'Assurance Maladie-Invalidité; Kas der Geneeskundige Verzorging van de NMBS Holding/Caisse des Soins de santé de la SNCB Holding

Themes of the interview guideline for members of the College

Focus was put on the tasks – the objectives – the budget of the SSF, the internal procedures followed within the health insurance funds, the functioning of the College of Medical Directors and the different decision mechanisms within the SSF, the definition and the use of criteria by the SSF, the existence of quantitative data related to the SSF, the relations with external stakeholders and the knowledge on comparable SSF mechanisms in foreign countries.

4.1.2.5 Analysis

The interview results were described and summarized. No in dept analysis was performed because of the explorative goal of this part of the report.

4.1.2.6 Limitations of the methodology

The most important limitations of the methodology used are:

- All interviewed persons are directly involved in the functioning of the SSF through their management/administrative function or as member of the College of Medical Directors.
- The views of the members of the College do not necessary represent the view of the health insurance fund they belong to. The interviews were executed from the perspective of their membership of the College of Medical Directors.
- To promote openness during the interviews, the results are presented in a consolidated anonymous way. Consequently no link is made between statements and individuals.
- Nor the transcript of the interviews nor the summary of the findings were submitted to interviewees for validation

4.1.3 Quantitative approach by administrative data analyses

The SSF maintains a database containing a multitude of information on the demands submitted to the SSF and their trajectory from introduction to final decision. Table 1 summarizes the information available in this database.

Table 1: Information available in the SSF database

Type	Subtype	Information available	Remarks
Patient characteristics		language	
		gender	
		sickness fund	national level
		nationality	
		type of employment	self-employed or not
Demand characteristics	timing	date of reception SSF	
		date of meeting SSF college	
		date of decision	
		date of notification of the decision	
	demand type	type of demand	original, renewal, revision[9], other
		origin of type of demand	domestic or not
	decision	what decision was taken?	
		who took the decision	SSF physician, SSF board, medical advisor local sickness fund
		reason for the decision taken	Insufficiently detailed. For rejected applications, only Belgium-abroad is available. For accepted applications, only subdivision of article 25 is available, no motivation to the applicability of the legal criteria was available
	prescriber	prescriber NIHDI identification	
		profession of prescriber	physician or not
		medical specialty of prescriber	
		Hospital NIHDI identification	
	treatment	ICD9-CM classification	
		product	Registered in a non-standardized way: multiple different values for a similar product or treatment
		duration	
		number of products	both demanded and attributed
		unit price	both demanded and attributed
		medical costs	both demanded and attributed
		relocation costs	both demanded and attributed
		hotel costs	both demanded and attributed
		location	domestic or not

9 The SSF reimbursement is often limited to a percentage of the cost till a fixed price is agreed on and the drug is transferred to the obligatory health care Insurance. The reimbursed amount is revised when the price is fixed.

We obtained the authorization of the Belgian Privacy Commission[14] to study a sample of this database. The sample consisted of the detailed information (see table 1) available on all demands of patients for the years 2004-2008 (for which an informed consent[10] was obtained 687 patients out of 4989 refused to participate).

The data at our disposal are used to describe the functioning of the SSF in terms of number of demands, number of patients, the reimbursement demanded and attributed per year, patient characteristics and demand characteristics by means of univariate and multivariate descriptive statistics and graphs.

Data were prepared and analyzed using Microsoft Excel 2007, SAS 9.1.3 and R 2.10.0

The SSF database was converted into a format suitable for the analyses planned in the present study. In general, the following transformations to the data were applied:

- exclusion of variables for which all values were missing:
  - was there a prior demand?
  - was additional information requested from the sickness fund?
  - NIHDI nomenclature code
  - hotel costs of the titular of the person entitled
  - advice requested and received from the Drug Reimbursement Commission (DRC/CTG/CRM)
- exclusion of 10 patients from the database for which demand characteristics were available, but not patient characteristics
- standardization of variables into first normal form (same content has the same value):
  - country of residence: deduced from free text field
  - decision: conversion of one variable into five different variables: decision taken, reason for decision, decision for treatments prior to change of legal criteria of 2005, treatment location, demand on principle[11].
  - Treatment group

The availability of the SSF annual reports made it possible to assess the representativeness of the sample we obtained. In these annual reports, statistics per year for several variables also in the SSF sample are available. The definition of "year" in the annual reports either means the year when the demand was introduced or the year when the SSF decision was taken. In the descriptive statistics that follow, we will mark the former as "introduction date" and the latter as "decision date".

A detailed description of the representativeness of the sample can be found in annex 9.3. A sample representing largely between 55% and 75% (depending on year and subdivision) of the demands introduced at the SSF between 2004 and 2008 was obtained. However, it does not constitute a random sample due to the informed consent. Moreover, the representativeness of the SSF sample is not always similar across year and subdivisions. This should be kept in mind when interpreting the results of the analyses on the SSF sample.

10 The "Sectoraal comité van de sociale zekerheid en van de gezondheid/ Comité sectoriel de la Sécurité Sociale et de la Santé" judged that it was necessary to inform the patient on the aims of the study and the use of the data of the patient's file. An information letter (see annex 9.2.) was drawn up by the KCE and sent to the patients by the NIHDI. Patients were allowed to refuse that there files were analysed (opting-out procedure).

11 The Board of the SSF can also decide on the reimbursement relying on an outline of the costs. In this case the respective sickness fund transfers a round up of the amounts that were paid in execution of the SSF decision. In that case the reimbursed amount is decided by the medical advisor of the sickness fund. The sickness fund will reimburse this amount within a delay of 20 working days after reception of the invoice.

For further analysis, 5 applications were excluded from the SSF because the decision was missing, and a further 501 were excluded because the decision was postponed (signalling an incomplete application) or was unclear, leaving 8223 (94.20%) applications of 4165 patients (96.8%) available between 2004 and 2008 (the year attributed to a demand was the year the decision was taken).

4.2 EVALUATION OF THE FUNCTIONING OF THE SSF

As indicated in the previous chapter (see sub-questions), the evaluation of the functioning of the SSF focuses on the explicit and implicit criteria used to assess eligibility for reimbursement of costs (e.g. what is an expensive treatment, what is a rare indication, what is a vital function), the clarity of these criteria for all parties involved, the channels by which stakeholders are informed on the existence of the SSF and the applicable procedures and last but not least the evaluation of the application procedure and decision-making process by all parties concerned (e.g. clarity of criteria, duration, customer friendliness).

4.2.1 Objectives of the qualitative survey

The aims of this qualitative survey are to obtain a global overview of the perception of the prescribing medical doctors, the social services and patients' associations on:

- Context of the SSF (number of SSF applications, number of patients involved in SSF submissions);
- The way they have been informed on the SSF existence and functioning;
- The need of a financial safety net for expensive medical treatments that are not reimbursed by the compulsory health insurance system;
- The objectives of the SSF, the degree to which these objectives are met and the main reasons for not achieving the set objectives;
- The other strategies or the other channels but the SSF they use to obtain financial support for costs of treatments to patients with rare diseases, rare indications or expensive treatments that are not covered by the compulsory health insurance system;
- The pertinence (is the SSF the right thing to do) and effectiveness of the SSF;
- Evaluation of the current intervention domains of the SSF and potential future intervention domains;
- The functioning of the SSF and particularly the implication for the respondents (procedure, criteria for intervention of the SSF, availability of the information, appeal possibilities, communication process, positive and negative elements).

4.2.2 Methodology of the qualitative survey

4.2.2.1 Population

In order to have a global overview of the perceptions on the SSF, stakeholders involved in the decision phase on requests for reimbursement at the SSF, i.e. the leading medical officer of the SSF and 6 medical directors of the health insurance funds (at national level) were interviewed (see sampling description of the functioning of the SSF 4.1.2.3).

In addition, stakeholders involved in the submission phase of the application for reimbursement i.e. patients, prescribing physicians, hospital social services were interviewed. Finally representatives of the pharmaceutical industry were interviewed.

The local sickness funds who act as the first step in the submission process were not interviewed because of the poor added value expected.

4.2.2.2 *Selection of the method*

Since the study objectives aims to explore the subjective view of stakeholders, we opted for a qualitative approach. For practical reasons, we chose to perform individual semi-structured interviews. Indeed, it was not possible to bring professionals, and particularly medical specialists, together in the same place at the same time to interview them by a focus group, while this data collection configuration should have been more productive because of the group dynamic and the brainstorming it creates. Moreover, qualitative research by focus groups would have required interviews of 6 to 12 persons. These have to be 'homogenous', i.e. to present the same characteristics regarding our segmentation criteria. We also would have needed at least one group of each type of respondents (heterogeneity of the groups) to have a global image of the representations of our population.

The advantage of semi-structured interviews is the flexibility, allowing new questions to be brought up during the interview as a result of what the interviewee says. The set of questions are prepared (clear focus for the interviewer on the themes) but open, allowing the interviewees to express opinions through discussion.

4.2.2.3 *Sampling*

Social services

We built a stratified purposive theoretical sample of social services based on the NIHDI individual data (for which patients have given their informed consent to be included in the database to analyze – see annex 9.2.).

As particularly social services that have been involved in the SSF procedure have sufficient experience with the SSF to judge the functioning, the pertinence, the effectiveness and the user-linked evaluation aspects, social services of prescribing hospitals[12] that have introduced at least 10 applications for reimbursement to the SSF in between 2004 and 2008 were considered. Twelve representatives of social services were interviewed.

Here also, the purpose of the sampling is to increase *credibility,* not to foster representativeness in the statistical meaning. We aim thus to maximise the different types of information that can be furnished by a sample of respondents including the full range of possibilities.

The segmentation criteria of this stakeholder population is described in annex 9.4.

Prescribing physicians[13]

We built a stratified purposive theoretical sample of prescribing physicians.

As particularly physicians who have been involved in the SSF procedure have sufficient experience with the SSF to judge the functioning, the pertinence, the effectiveness and the user-linked evaluation aspects, physicians that introduced at least 10 applications for reimbursement to the SSF in 2006 and 2007 were considered. The number of applications does not necessarily reflect the number of patients concerned (one application could be a renewal of the prescription, or several separate treatments for the same patient(s)). Thirteen physicians were interviewed. The purpose of the sampling is to increase *credibility,* not to foster representativeness in the statistical meaning. The segmentation criteria of the sample of prescribing physicians is described in annex 9.5.

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12 We define 'prescribing hospital' as the hospitals in which physicians have prescribed a treatment for which an application for an intervention of the SSF was introduced to the SSF

13 We define 'prescribing physicians' as the physicians who have prescribed a treatment for which an application for an intervention of the SSF was introduced to the SSF

Patient organizations

To select Belgian patient organizations to be contacted for the qualitative research, we started with Rare Disease Organization Belgium (Radiorg.be), established in January 2008 and recognized by Eurordis. Radiorg.be, the Belgian alliance for rare diseases is an umbrella organization, representing more than 80 Belgian rare disease organizations (http://www.radiog.be).

To further extent the number of patient organizations we used the following criteria:

- The patient organization has to be known at international level. We limit ourselves to the members of the European organization for rare diseases.
- The patient organizations represent a variety of SSF submissions.
  - The decentralized SSF procedure

    Since 2001 the decentralized SSF procedure is applicable to patients with EB (Epidermolysis Bullosa). The treatments reimbursed via the SSF procedure are mostly ointments, bandages,… Therefore the decision was taken to include their patient organization, Debra Belgium.
  - The centralized procedure

    One patient organization needed to represent patients for whom the treatment was mainly focused on expensive drugs. Therefore we included PH Belgium, the patient organization for pulmonary hypertension. SSF files are mostly related to the reimbursement of the very expensive drugs Remodulin® and/or Flolan® .

    One patient organization needed to focus on children. BOKS, the patient organization for adults and children with metabolic diseases has a clear focus on children as more then 80% of their families/members are linked to illness of children.

    As a last patient organization, we included NEMA, the patient organization for neuromuscular diseases, as the organization covers a huge group of rare diseases and a neurological focus was not yet realized via the other patient organizations.

The 5 selected patient organizations were contacted by phone to ask for their collaboration. All organizations confirmed their participation and meetings were scheduled.

The initial idea to include also patient organizations representing rare diseases, often confronted with a rejection of SSF submissions was not possible. The necessary data to identify and select these patient organizations could not be extracted from the SSF database.

Pharmaceutical industry representatives

During the project execution the question was raised to include representatives of the pharmaceutical industry in the qualitative data collection. The project team contacted pharma.be, the Belgian pharmaceutical industry organization, who distributed an email to the members of their "orphan drug working group" as these member companies are often confronted with the SSF. Thirteen companies reacted positively (prepared to cooperate by means of an interview) of which 4 indicated they had never or seldom experience with the SSF. Due to time restrictions, it was decided to meet a small number of representatives of the pharmaceutical industry. From the remaining 9 companies, the project team selected 4 companies, who were contacted to schedule a telephone interview. This selection was made following the advice of the general adjunct director of pharma.be, who indicated the companies that are most familiar with the SSF thanks to their professional experience or because they produce orphans or cancer drugs for which requests to the SSF are frequently introduced.

The interview topics to be covered are: reimbursement agreements between the NIHDI and pharmaceutical companies, development and registration of new drugs, specific problems related to drugs for rare diseases, off label use of drugs for rare diseases, compassionate use programs and medical urgency need programs and their view on the SSF (conceptual and operational).

We also received a general comment on the SSF from Pharma.be, the Belgian organization of the pharmaceutical industry.

4.2.2.4 Data collection

Tool

We used a semi-structured interview guideline, specific for each kind of respondent, consisting of a number of orienting generic questions and a number of sub-questions in order to clarify or expand the view of the interviewee has been elaborated and discussed by the project team.

Questions aim to respond to the objectives of the survey.

It was tested in two interviews and, in function of the results, further adjusted. The interview guidelines are included in annex 9.7., 9.8. and 9.9. to this report.

Interview processing

Each interview was conducted by one or two persons. When the interview was conducted by two persons, one person leaded and moderated the interview while the other person took notes and supported the moderator when issues needed further elaboration. Additionally, the interviews were digitally recorded with the consent of the respondents (all respondents consented). The recordings were destroyed after processing. The interviews were conducted by the external consultants and took place in the native language of the respondents.

Following the interview, both the moderator of the interview and the assisting researcher wrote down their individual preliminary impressions on the interview in short debriefing notes.

Secondly, the digital record was used to perform a thorough analysis. The researchers developed and filled up a grid, in which the interviews were analyzed at a descriptive level: a descriptive account of what was said (by whom) related to particular topics and questions. In this grid, the unit of analysis were the respondents and the units of record the questions or topics.

Based on the notes taken during the interview and the grid developed after the interview, the note taker made a draft non-literal transcript. The moderator complemented this draft and added additional information. Unclear aspects or issues were discussed by both researchers. Findings were illustrated by several quotations. Confidentiality was respected because no idea or result should be related to nominative persons.

4.2.3 Quantitative approach: the SSF sample

The SSF sample data were used to illustrate where possible the description of the SSF by means of univariate and multivariate descriptive statistics and graphs. As the SSF sample available to the current study has certain limitations (as discussed above), a hypothesis testing statistical approach to assess the propositions found in the qualitative survey was not performed.

4.3 EXPLORATION OF (COMPARABLE) SAFETY NETS IN A SELECTION OF FOREIGN COUNTRIES

4.3.1 Objectives

The third research question focuses on the existence of comparable safety nets for cost of treatment of rare diseases or rare indications in a selection of foreign countries. The objective of this research question is twofold:

- Are there "mirror institutions" of the SSF or comparable safety nets abroad?
- How is the care of selected cases of the targeted SSF population organized in the selected countries?

4.3.2 Methodology

4.3.2.1 Selection of countries

The international benchmark exercise focuses on France, the Netherlands and Spain. The common characteristics of these countries leading to this selection were comparable health care systems (the public share of total expenditure on health represent at least 66%)[15], comparable living standards and their geographical proximity to Belgium.

In addition to these common elements, the following aspects have contributed to the final selection:

- The Netherlands
  - The changes in the health provision and insurance system introduced in 2005, leading to a further liberalisation of the health care system;
  - The existence of a society for rare diseases (stichting zeldzame ziekten fonds) - http://www.zzf.nl/.
- France:
  - The existence of a national plan for rare diseases (2005-2008) with 10 strategic priorities established in 2004 http://www.eurordis.org/IMG/pdf/EN_french_rare_disease_plan.pdf).
  - The European Commission's public health programmes attached much importance to the rare diseases. Many projects on rare diseases haven been funded and important international networks at EU level have been created. For rare diseases the ORPHANET and EURORDIS are by far the most elaborated network on rare diseases. Both are French initiatives
    - Eurordis, the European Organization for rare diseases is a patient driven alliance of patient organizations and individuals active in the field of rare diseases. www.eurordis.org
    - Orphanet, the portal for rare diseases and orphan drugs. http://www.orpha.net/consor/cgi-bin/index.php
  - The existence of GIS, a national institute for rare diseases (institute des maladies rares). http://www.institutmaladiesrares.net
- Spain
  - The existence of FEDER, the Spanish federation for rare diseases (federacion Espanola de enfermedade rares) http://www.enfermedades-raras.org/es/default.htm
  - The existence of IIER, the research institute for rare diseases (the instituto de Investigacion de enfermedades raras) - http://iier.isciii.es/er/

4.3.2.2 Review of websites

The websites of the ministries of health and the website of institutes and federations already identified for the selected countries (see previous paragraph) were reviewed.

Based on this approach we made a list of institutions/stakeholders and experts to contact.

4.3.2.3 List of actual cases

During our interviews with the leading medical officer of the SSF and the 6 members of the College of Medical directors of the SSF, the question was raised if they were aware of comparable systems in foreign countries. No one was aware of the existence of a "mirror organisation" or a comparable safety net system abroad.

As a consequence we did foresee an alternative approach, to increase the chance of receiving answers to the specific research questions related to the international benchmark.

The research team developed a draft list of case studies (comprising drugs, implants, treatments for which reimbursement is asked for at the SSF) and the same exercise was done by the leading medical officer of the SSF. During a meeting both draft lists were compared and consolidated into one list. The cases were translated in English and a final check on the medical terminology was performed by the leading medical officer of the SSF. The list of cases is included as annex 9.17.

The SSF cases represent a wide variety of real life SSF applications. The following criteria were used to include cases in the final list (non cumulative criteria):

- Cases represented common SSF applications (frequent SSF submission);
- Cases represented a significant proportion of the budget (relatively costs per SSF application);
- Cases covered the different SSF categories;
- Cases included on specific request of the SSF (cases for which the SSF wanted to know how these were reimbursed abroad, cases for which discussions have taken place, …).

4.3.2.4 Data collection

For the three countries, we contacted the persons identified through the web search by telephone. Based on their feedback and suggestions, we contacted other persons for an interview, red documents and legislation, …

As these aspects differ substantially between the three countries, the different sources used are specifically mentioned in each country paragraph.

Desk research and telephone interviews were the most common ways to collect the data in the three countries.

4.3.2.5 Treatment of results

General description of the reimbursement system for rare diseases in respective country and when possible overview of how the SSF cases are handled in the three countries.

5 DESCRIPTION OF THE FUNCTIONING OF THE SSF

5.1 BUDGET OF THE SSF

The annual budget of the SSF consists of a fixed amount. This amount is set each year by a Royal Decree and taken from the global NIHDI budget.

5.1.1 Actors and steps of the formal budget cycle

The financial services of the NIHDI are the initiators, calculating and proposing a first draft budget. This draft budget is calculated on the basis of semesterial audit reports from the actuarial services of the NIHDI and its last accounting data. These are compared with the expenses from the Sickness funds and with the amounts reflecting the engagements taken by the 'College of medical directors'. These technical calculations start up during the month of May.

The different NIHDI commissions or committees are asked to provide an inventory on the needs to be covered. For the SSF the 'College of medical directors' can, if deemed necessary, propose changes to the draft budget based on 'new needs' detected. These proposals have to be transmitted to the actuarial services of the NIHDI before the end of June.

In actual practice, the SSF the 'College of medical directors' does not really provide such inventory of 'open needs' nor proposes 'new needs ' to be met. Instead the actuarial services discuss the draft budget with the leading medical officer of the SSF. On the basis of expected changes in the SSF-regulation or of new initiatives, products or treatments expected to be reimbursed in the next budget period, the draft budget is being adjusted.

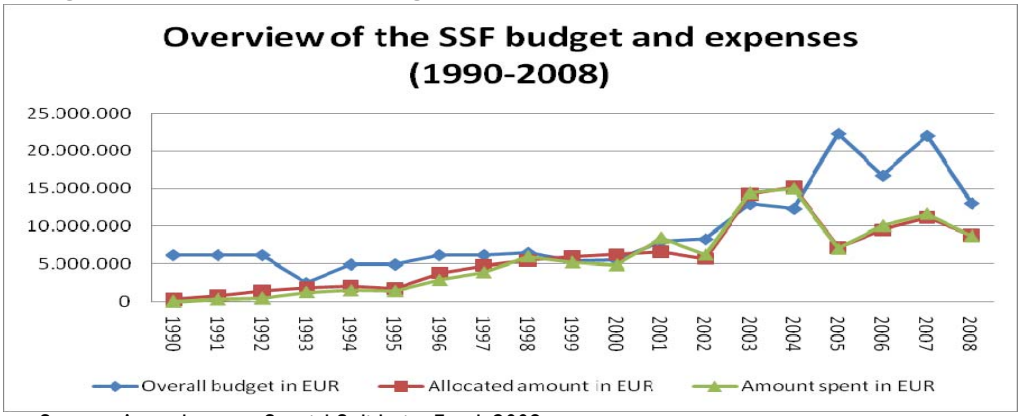
The overall draft budget for the NIHDI is discussed at the level of the 'Insurance committee' of the NIHDI during the month of July. Before the 15th of September the NIHDI 'Budget control commission' eventually proposes measures to alter the global NIHDI budget in view of the global budget objectives. Before the 1st of October the draft budget is actualized by the actuarial services integrating the financial data from the first 5 months of the current year. The global budget is finally adjusted by the NIHDI 'Insurance committee' in order to meet the global budget objective (maximum + 4,5% with respect to the previous year plus index). Finally the budget is formally fixed by the 'General Council', presented to the Federal Minister of Social Affairs and submitted to the approval of the Federal Government. The amount allocated to the SSF, as a part of the global NIHDI budget, is formally fixed by a Royal Decree.

In fact the SSF budget is a black box where expenses depend on individual demands from patients and individual decisions from the 'College of medical directors' on these demands. The total expenses financed by the SSF indirectly are influenced by decisions taken outside the SSF. If costs of medical treatment, previously reimbursed by the SSF are accepted and integrated in the compulsory health insurance system, this actually means the SSF does not longer reimburse them. Such decisions thus have direct consequences for the SSF expenses. If a drug is recognized as an orphan drug or a demand for approval is being rejected by the 'College on Orphan drugs', the SSF can no longer reimburse the costs of these products. In that scope prediction of future costs and expenses for the SSF is difficult.

5.1.2 Budget evolutions

The following chart (figure 1) gives an overview of the budget of the SSF since the start-up in 1990.

Figure 1: Overview of the budget of the SSF since 1990



Source: Annual report Special Solidarity Fund, 2008

The budget of the SSF varies substantially over the years. The chart shows an important increase of the budget for 2003 compared to the previous years. The budget increased as a consequence of the changes in the criteria for reimbursement for the additional costs of children with chronic diseases. In 2005 a more fundamental change on the SSF regulation took place with changes to the reimbursement of costs of exceptional medical treatments, the children with chronic diseases and the cost of medical treatments abroad as described in article 25 of the coordinated law of July 14th, 1994 on the insurance of medical treatment and allocations.[12] Anticipating on the effects of these changes, the SSF budget substantially increased from €12.371.000 in 2004 to €22.377.000 in 2005 (art. 2 Law 27/04/2005). [16] However, real expenses fell from €14.998.593 in 2004 to €7.031.980 in 2005. The predicted impact of the legal reform did not show. In fact for the year 2005 the number of patients that were reimbursed, diminished from a number of 1.674 in 2004 to 1.525 in 2005. Applications fell from 2.682 in 2004 to 2.218 in 2005. For the year 2005 expenses related to the chronically ill children, calculated from the decisions taken that year by the 'College of medical directors' reach the amount of only €94.272 representing 34 patients. The expected raise of these costs only (partially) occurred in 2006. The main reason for the decline in expenses was the acceptance of 4 drugs as 'Orphan drugs', (Tracleer®, Fabrazyme®, Replagal® and Aldurazyme®) resulting in a transfer of the corresponding expenses to the compulsory health insurance system and a non-reimbursement of these costs by the SSF from then on. The budget increase of 2005 to 2007 was diminished in 2008 to €13.102.00. Real expenses substantially decreased in 2008 merely due to the fact that a number of products and interventions have been accepted for coverage by the compulsory health insurance.

The difference between the budget and the actual expenses was substantial in the start-up period of the SSF. Since the SSF was completely new and no historical data where available, such differences can be seen as normal. For the year 2001 the actual expenses exceeded by little the budget. For 2003 and 2004 the budget was exceeded substantially. For 2003 the difference was of €1.413.593 or 10,86 %. For 2004 the difference reached a much higher level at €2.627.593 or 21,24 %.

Strictly an excess of the budget is not allowed. For NIHDI expenses the excesses have to be compensated by taking correcting measures. For the SSF such correcting measures are not feasible since reimbursement is an absolute right for the patient when meeting the criteria as foreseen in the corresponding SSF regulation.

When the budget is being exceeded, the 'College of medical directors' has to ask explicit permission to the NIHDI 'Insurance Committee' to be allowed to make further payments to the patients. This has never caused any problems.[14]

The SSF can limit the reimbursement to a percentage of the total cost. Mostly a percentage of 60% or 75% is granted . If a percentage of 75 % is granted the patient's share can be limited to an amount varying between €1000 and €1500 on annual basis. In case the SSF grants 60% of reimbursement, there is no maximum annual personal share.

In 2006 and 2007 the SSF budget was calculated expecting the reimbursement of two specific drugs, Flolan® and Remodulin® by the compulsory health insurance system. Such reimbursement under the compulsory health care insurance system would have resulted in a revision of previous decisions by the SSF where only 60% of the costs of these medicines were reimbursed into a reimbursement at 100%.[15] However, the surplus expenses did not occur in 2006 or 2007 since the NIHDI 'Commission of reimbursements of medicines' did not decide upon reimbursement. As a consequence, the amount that has been assigned for these reimbursements was not spent and the SSF budget reported a substantial budget surplus. For 2006 the surplus was of €6.692.598 or 39,91 %. For 2007 the surplus reached €10.428.286 or 47,21 %.

5.1.3 Real expenses versus amounts attributed

When looking at the expenses of the SSF one has to notice the expenses are the amounts actually paid by the health insurance during the budget period. The amounts granted are the allocated amounts based on the outlines of the costs. Sometimes the real expenses however do not perfectly fit with the outlines. This explains the differences between the granted amounts and the paid amounts. In table 2 below we give an overview of the actual budget, the financial effect of the decisions taken by the 'College of medical directors' and the actual real payments executed during the correspondent year. One can notice the respective differences that can be substantial.

Table 2: SSF Budget versus amounts granted and amounts paid since 1990

Year	Budget (€)	Amounts granted (€)	Amounts paid (€)
1990	6.197.338	309.020	7.210
1991	6.197.338	767.080	321.838
1992	6.197.338	1.358.650	442.603
1993	2.478.935	1.837.150	1.211.662
1994	4.957.870	2.053.430	1.455.801
1995	4.957.870	1.704.630	1.363.069
1996	6.197.338	3.816.470	2.854.003
1997	6.197.338	4.802.780	3.889.873
1998	6.502.247	5.488.460	5.981.501
1999	5.453.658	5.974.000	5.248.924
2000	5.480.926	6.253.240	4.860.508
2001	8.061.497	6.600.120	8.477.758
2002	8.317.000	5.685.120	6.226.380
2003	13.017.000	14.235.080	14.430.593
2004	12.371.000	15.252.240	14.998.593
2005	22.377.000	7.184.528	7.031.980
2006	16.769.000	9.510.977	10.076.402
2007	22.090.000	11.205.651	11.661.714
2008	13.102.000	8.826.009	8.692.000

Source: Annual report Special Solidarity Fund 2008

14 Personal communication responsible for the budget follow up within the NIHDI.

15 In order to abstain from any inference in price discussions with the FOD Economie, KMO, Middenstand en Energie/ SPF Economie, P.M.E., Classes moyennes et Energie, the SSF often allows an advance of 60% instead of full reimbursement.

5.1.4 Spending patterns

As indicated in the table below (table 3), the medical costs represent the majority of the global SSF amounts granted. For 2008 they reach over 98% of the total expenditure. The rise of expenses for reimbursement of extra costs for "chronically ill children" is substantial. They represent 5% of total SSF expenses in 2007 and even 11% 2008. Since 2005, the SSF registers the expenses resulting from revisions of SSF decisions by the labour courts. The SSF procedures foresee the right for the patients to appeal any decision taken by the SSF. The cases are ruled by the labour courts. Although the number of cases decreases over the years, the costs assigned following a court ruling steeply rose in 2008 to €427.285. In 2008, the costs for medical treatment abroad were very limited, yet the number of patients having introduced a request for reimbursement remained stationary.

Table 3: SSF Amounts granted 2003-2008

EUR	2003	2004	2005	2006	2007	2008
medical costs (§ 2+EB (3) +art. 25bis, ter § 1, ter § 2, quater)	14.169.225	14.944.229	6.977.852	8.969.777	9.853.777	7.086.419
travel costs (§ 4+ art. 25sexies)	19.107	20.593	12.593	6.588	9.671	10.295
costs of stay (§ 4+ art. 25sexies)	2.757	7.585	4.664	15.023	6.419	6.593
medical costs (§ 4+ art. 25sexies)	45.889	275.000	4.338	27.790	37.164	430
« chronically ill children » (§ 3 en artikel 25quinquies)	0	4.833	94.272	329.039	525.626	952.708
subtotal	14.236.978	15.252.240	7.093.719	9.348.217	10.432.657	8.056.445
Costs rulings labor courts	-2	-2	90.808	162.760	112.533	427.286
Total amount spent	14.236.978	15.252.240	7.184.528	9.510.977	10.545.190	8.483.731
Decisions delegated to the health insurance funds			288.741	797.118	660.461	342.278

Source: Annual reports Special Solidarity Fund, 2003, 2004, 2005, 2006, 2007, 2008

Below we give an overview of the SSF activities between 2004 and 2008, using the following indicators: amount spent/type of treatment (table 4), number of patients/type of treatment (table 5), average amount spent/type of treatment/patient (table 6).

The most important expenses for the SSF are the reimbursements to the patients of the costs of drugs (in 2008: 92% of total amount allocated for 67% of total number of patients requested for reimbursement). These expenses for drugs vary substantially from 2003 to 2008. In 2007 however, the expensive orphan drug Myozyme® was adopted in the chapter IV of the list of reimbursable drugs. This was translated in a drop of expenses by the SSF for drugs in 2008.

Table 4: Reimbursement in €/per type of treatment 2004-2008

Agreements	Reimbursement in € / per type of treatment				
	2004	2005	2006	2007	2008
Medicines	12.935.507	5.339.552	8.031.943	10.323.674	7.970.122
Implants	940.801	957.047	555.777	243.250	305.573
Instruments, prothesis, orthesis	124.111	229.029	135.408	140.528	116.358
Food for particular nutrition uses	24.876	229.029	27.255	10.507	18.338
Practictioner's fees	0	0	7442	107	5.338
Medical Techniques	518.668	182.931	155.097	68.424	49.699
Ointments, medicines, bandages… for skin diseases	395.770	332.934	510.976	324.067	241.034
Bandages, tampons, protection material…	4.496	1.680	9.918	15.918	2.696
Following a treatment plan		6.375	24342	22026	32.025
Total	14.944.229	7.072.124	9.458.158	11.148.501	8.741.184

Source: Annual reports of the Special Solidarity Fund 2004, 2005, 2006, 2007, 2008

The total number of patients reimbursed by the SSF decreased drastically from 1.609 patients in 2004 towards 637 patients in 2008.

The decrease in number of patients in the category "implants" and "medical treatments" explains the overall decrease in number of patients.

Table 5: Number of patients per type of treatment 2004-2008

Agreements	Number of patients (1)				
	2004	2005	2006	2007	2008
Medicines	502	358	337	384	435
Implants	713	704	363	126	85
Instruments, prothesis, orthesis	45	38	54	44	37
Food for particular nutrition uses	13	19	22	6	9
Practictioner's fees	0	0	2	1	5
Medical Techniques	319	151	110	81	35
Ointments, medicines, bandages... for skin diseases	14	15	17	21	15
Bandages, tampons, protection material...	3	2	8	5	4
Following a treatment plan	0	2	9	14	12
Total	1.609	1.289	922	682	637

Source: Annual reports of the Special Solidarity Fund 2004, 2005, 2006, 2007, 2008

The average amount spent per category and per patient is high for "medicines", "ointments-medicines and bandages for skin diseases".

Between 2004 and 2007 the average amount spent per patient increased from €9.288 to €16.347 and decreased to €13.722 in 2008.

A possible explanation for the different rate in decline of number of applications compared to number of patients, is that in more recent years, the number of renewals have increased compared to the number of new applications.

Table 6: Average amount of reimbursement per patient per type of treatment 2004-2008

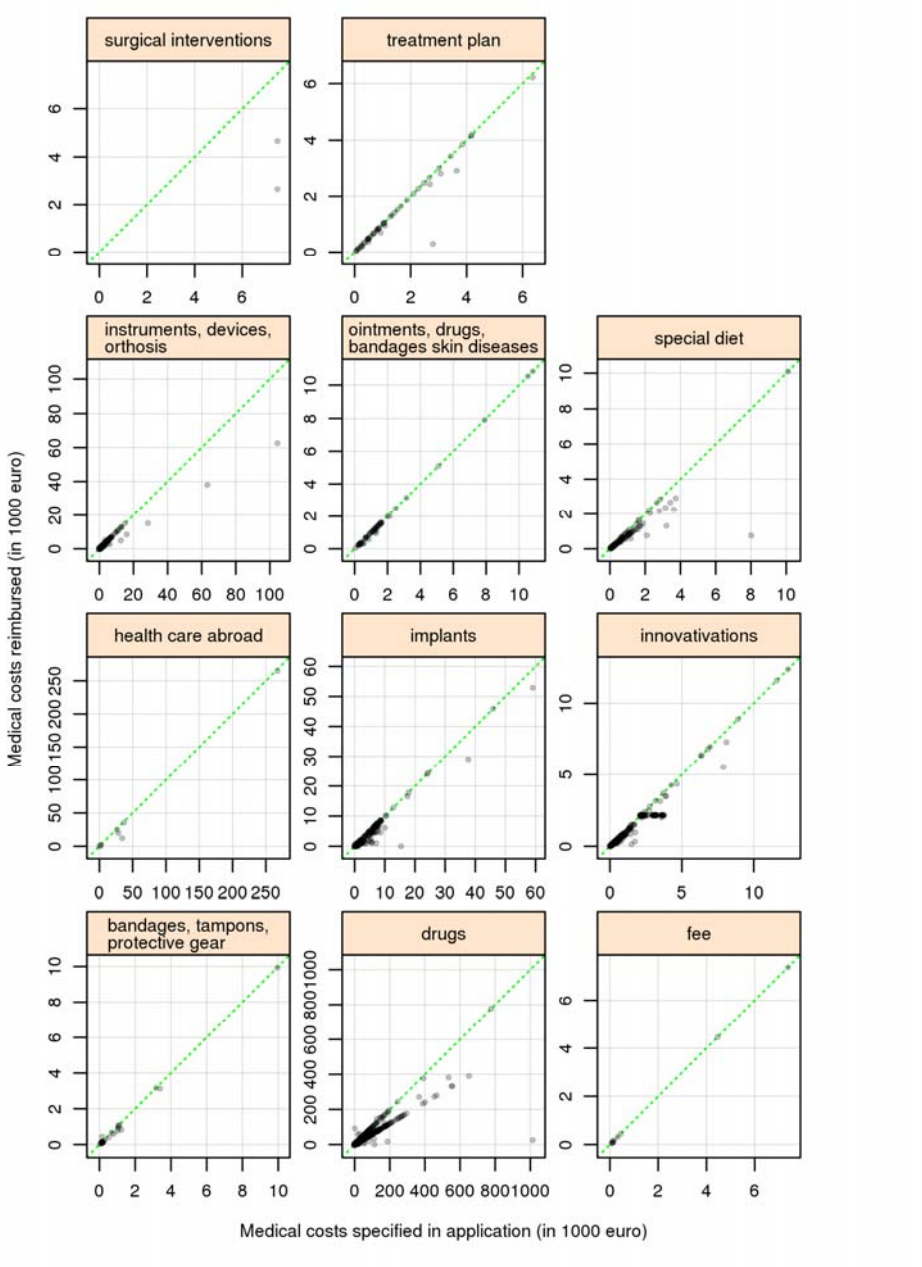
Agreements	Average amount of reimbursement / patiënt (2004-2008)				
	2004	2005	2006	2007	2008
Medicines	25.768	14.915	23.834	28.593	18.322
Implants	1.320	1.359	1.531	1.931	3.595
Instruments, prothesis, orthesis	2.758	6.027	2.508	3.194	3.145
Food for particular nutrition uses	1.914	12.054	1.239	1.751	2.038
Practictioner's fees	0	0	3721	107	1.068
Medical Techniques	1.626	1.211	1.410	845	1.420
Ointments, medicines, bandages... for skin diseases	28.269	22.196	30.057	15.432	16.069
Bandages, tampons, protection material...	1.499	840	1.240	3.184	674
Following a treatment plan	0	3.188	813	1.573	2.669
Total	9.288	5.487	10.240	16.347	13.722

Source: Annual reports of the Special Solidarity Fund 2004, 2005, 2006, 2007, 2008

As the SSF determines itself what part of the costs are reimbursed, differences are observed in the SSF sample between the amount of reimbursed asked and granted. This difference seems to be associated with the type of treatment applied for (see figure 2). In particular for drug treatments, most applications are reimbursed up to a certain amount: 41.6% of applications at 75% reimbursement, 13.8% at 85% reimbursement, and 13.3% at 60% reimbursement. In contrast, 20.8% of drug treatment applications are fully reimbursed. Similarly, 19.4% of special diet are fully reimbursed, while 35.2% are reimbursed at 75%, and 24.4% at 78%.

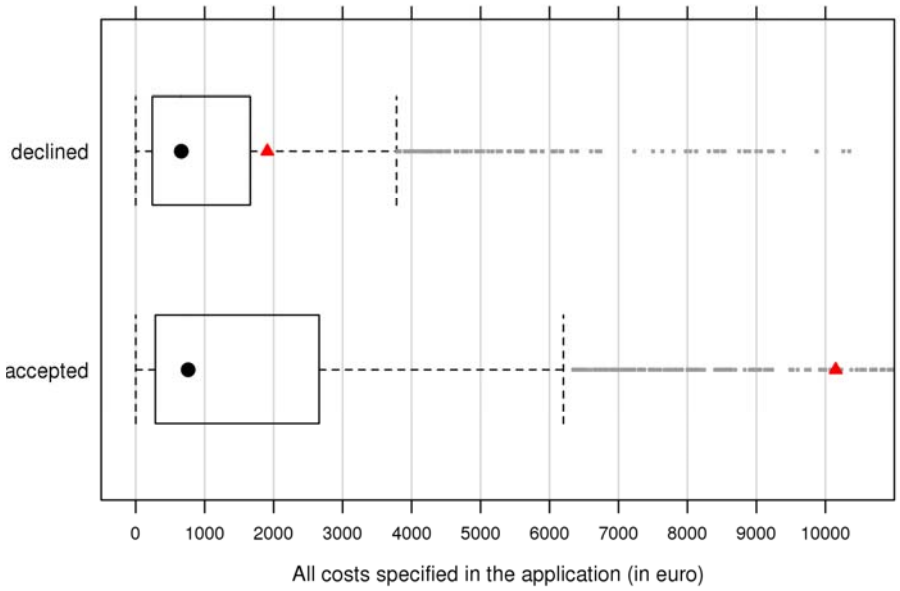
As can be seen in figure 2, in a small number of cases (0.7%), the actual reimbursement is larger than the reimbursement specified in the application. This can be explained by the fact that in these applications, the cost of a treatment is known only as an estimate which is smaller than the final cost of treatment.

Figure 2: Medical costs in the SSF sample as specified in the application versus as granted in function of treatment type (the dotted line corresponds to full reimbursement).



Accepted applications tend to have a more substantial amount of reimbursement specified in the application compared to declined applications, but declined applications are not by default less expensive than accepted applications (see figure 3). Of the 10% most expensive accepted applications, 1% is over €168.938, compared to €21.321 for declined applications.

Figure 3: All costs in the SSF sample as specified in the application in function of the decision taken (black dot is the median, the red triangle is the mean; the 10% most expensive applications are not shown).



5.2 PROCEDURE TO SUBMIT AN APPLICATION TO THE SSF FOR REIMBURSEMENT

5.2.1 Submitting an application for reimbursement

Every citizen has the obligation to adhere to one of the 7 health insurance funds, recognized by the Public health authority. Most of these health insurance funds are structured on a regional basis and are divided in local "sickness funds".

One of their tasks is to reimburse, under the supervision of the NIHDI, the costs of medical treatments and drugs covered under the compulsory health system.

Whenever a citizen has medical expenses for which the compulsory health system provides reimbursement, he has to enter the prescribed documents to his local sickness fund. If the expenses meet the conditions set out by the public health system, the sickness fund will reimburse the patient in accordance to the regulation and tariffs from the NIHDI.

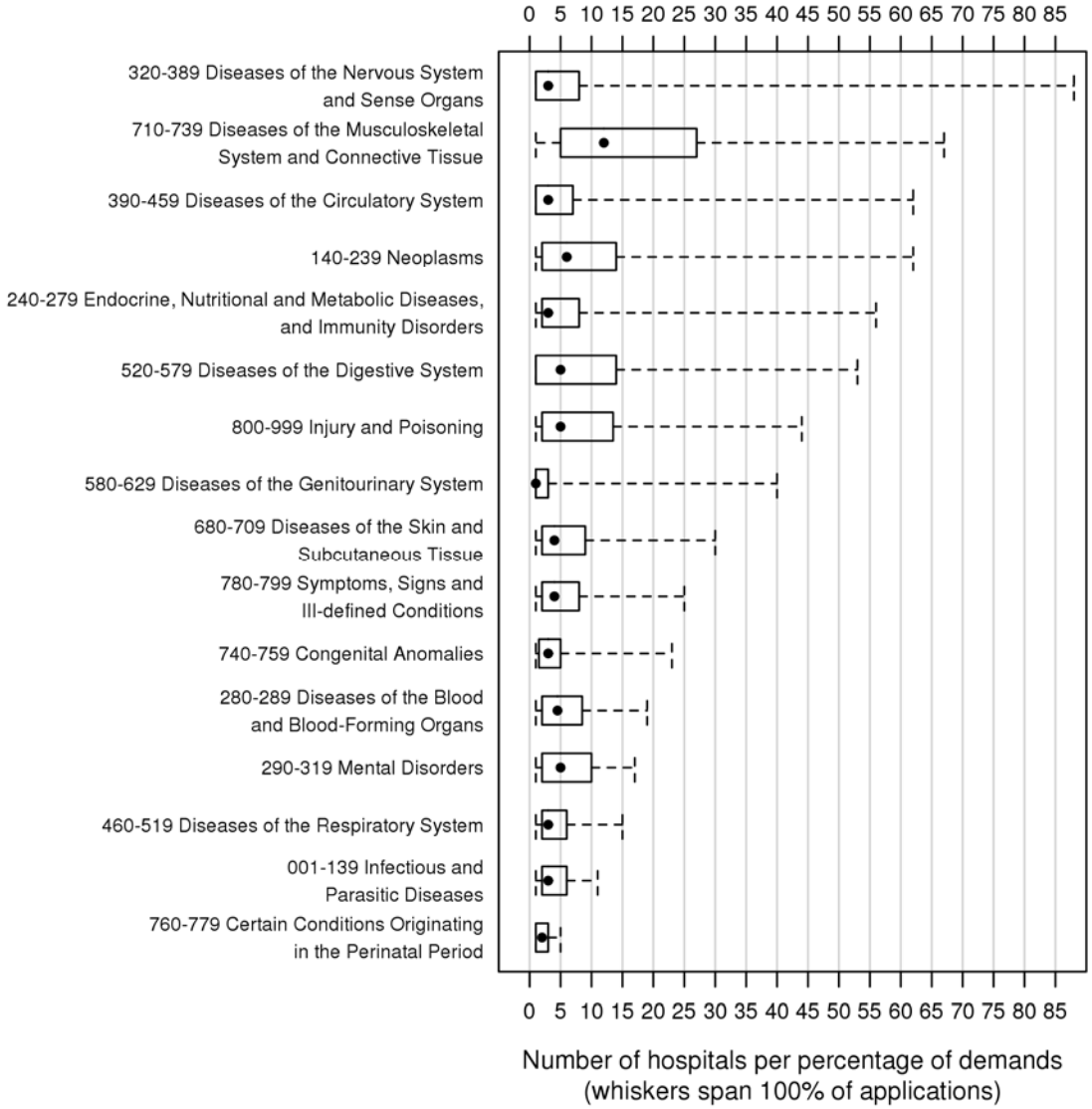
The same procedure is applicable for the requests for reimbursement of medical costs that are covered by the SSF. The initiative to submit an application for reimbursement of costs by the SSF has to be taken by the patient. The patient (or his representative) is solely entitled to apply for reimbursement entering a demand to the local sickness fund he adheres to. In order to be valid, the demand has to be introduced to the local sickness fund at the latest 3 years after the medical intervention (art. 25septies §2 in fine).

The local sickness funds mostly have social services that will help the patient (or his representative) to compose his file in order to be eligible for reimbursement. These local social services also inform and give advice to the patient on their rights and on how to complete the request for reimbursement.

For the majority of the applications, a hospital physician can be identified as the prescribing physician. In the SSF sample, for 69.1% of the applications, a hospital could be identified. Most applications arise from a small number of hospitals: 74.8% of all hospital related applications originate from 13 hospitals (7.9%), almost all linked to a university. A similar phenomenon is found when looking at ICD-9-CM diagnostic class: for most diagnostics classes, a limited amount of hospitals is responsible for the bulk of applications (see figure 4).

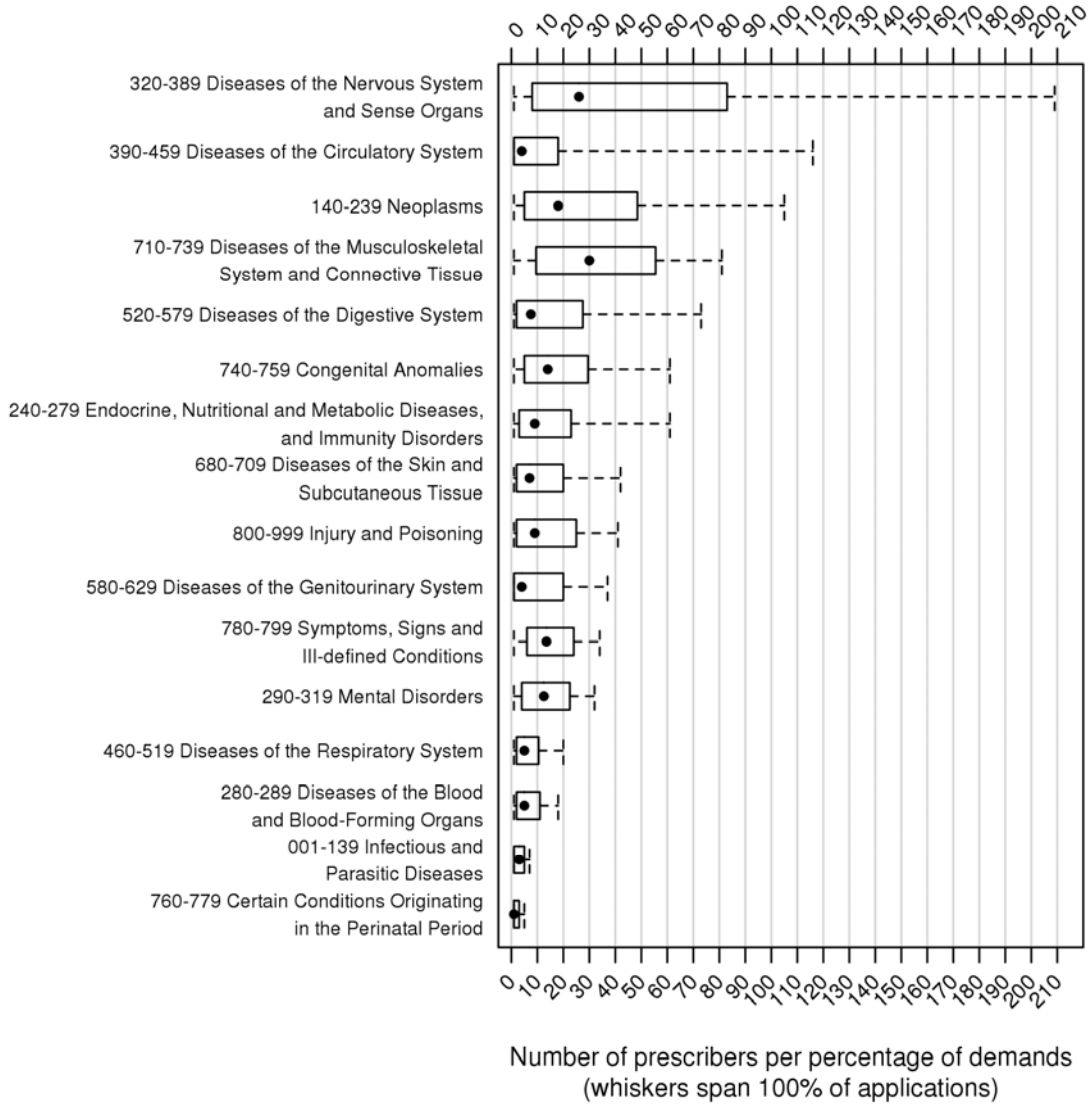
For example, for 320-389 Diseases of the Nervous system, 75% of all hospital related applications originated from 7 hospitals, while 90 different hospitals where the source for at least one application between 2004 and 2008.

Figure 4: Number of hospitals per percentage of applications they represent in the SSF sample in function of ICD-9-CM diagnostic class



Similarly, for the 31.9% of applications where the prescriber was not linked to a hospital in the SSF sample, most applications arise from a limited number of physicians (see figure 5). For example, 75% of all applications on 390-459 Diseases of the Circulatory System were prescribed by 18 physicians out of 116 physicians.

Figure 5: Number of prescribers per percentage of applications they represent in the SSF sample in function of ICD-9-CM diagnostic class.



5.2.2 Procedure at local sickness fund level

The patient submits "the application for reimbursement of costs by the SSF" to his local sickness fund. The sickness fund will be the only direct contact point for the patient. All information on the further treatment of his application will be transmitted to him by this local sickness fund which will – at the end – also inform the applicant on the decision taken by the SSF. The patient himself has no direct contact with the SSF services or with the other instances intervening in the administrative handling procedures of his request.

The SSF applications are treated in a comparable way in the different sickness funds. Since sickness funds differ in scale, it is obvious that the large-sized sickness funds have a more differentiated and specialized organizational structure.

Patients themselves are not deeply involved in the composition of their application file. They are asked to provide the necessary documents that are needed to enter a valid request for reimbursement. In most of the local sickness funds the patient will be directed towards the social service. These are mostly the administrative driver behind the further SSF procedure and are the main contact point for the patients.

The handling of the patient's request for reimbursement is divided in two different parts: The administrative handling and the handling of the medical aspects of his request.

The administrative part is taken care of by the social services of the local sickness funds. These services will verify if all necessary documents are available and all medical information has been entered by the patient. In order to complete the file, the social services will contact, if necessary, the health care provider (the prescribing medical doctor or the hospital where the patient is being treated).

Once the patient's file is complete, the administrative services of the local sickness fund will transfer the file to the medical department of the same local sickness fund. The medical department of local sickness funds is managed by the advising doctor(s).

The focus of the action at local sickness fund level lays merely on the administrative completeness of the application file, since this is a requirement to eligibility for reimbursement. Subject-related activities, such as research on the specific disease the patient is suffering from or indications, at the level of the local sickness fund are rare.

From the interviews with the medical doctors of the health insurance funds (at national level) we noticed local sickness funds have little or no standard guidelines to evaluate the validity of the patients' request for reimbursement.

At local level no specific selection criteria are taken into account. Once a demand for reimbursement has been entered by a patient, member of the sickness fund, the request will be handled and completed if necessary. The local sickness funds do not have the authority to refuse the settlement of a request for reimbursement of costs by the SSF. Even if the local sickness fund is clearly convinced the application of the patient does not meet the criteria set by the SSF and thus has no chance on being accepted for reimbursement, the local sickness fund still has to handle his request and enter it for decision by the SSF. In such a case the social services might draw the patient's attention on the expected refusal of reimbursement by the SSF, but at the end it is up to the patient to take the final decision on whether to apply for reimbursement or not.

One of the criteria for obtaining reimbursement of costs is that the applicant needs to have used all possible other rights to reimbursement by other (private of public) insurances he can benefit of. The local sickness fund will ask the patient to provide a declaration 'on honor' that he has no other personal rights on reimbursement and did not obtain a refund from any other private insurance and that all rights following Belgian or foreign legislation have been exhausted. In case another insurance only reimburses part of the cost, the patient has to explicit the amount eligible for reimbursement by this source. Next to the declaration 'on honor', the request for reimbursement needs to be composed of an information sheet, a prescription drawn up by a physician and a medical report allowing to check if the criteria of the respective reimbursement category are met and a detailed bill or an outline of the costs in case of a demand on principle.

After the administrative handling of the patients' request for reimbursement, the request will be transferred to the national level (the health insurance funds) for further treatment. Each file always includes a formal advice from the advising doctor of the local sickness fund on the validity of the request for reimbursement. Mostly this advice is focused and limited to the completeness of the application file.

5.2.3 Procedure at the national sickness funds

The applications for reimbursement of costs by the SSF entered at local sickness fund level are transmitted to the national sickness funds. The patient's application, the requested documents and the advice issued by the medical advising doctor of the local sickness fund are included into the application file.

At national level the applications are reviewed from an administrative and medical point of view.

At first they are treated by the administrative staff operating under the responsibility of a medical director. The number of administrative officers treating the patients' files differs, depending on the size of the national sickness fund. The larger sickness funds mostly have different administrative officers.

The administrative control merely concerns the completeness of the application file. If documents fail, or information is missing, the administrative staff will contact the local sickness fund to complete the file. Some national sickness funds use a self developed checklist that is based on the criteria for reimbursement set out by the SSF, others only use the official application form.

The application file has to be transferred to the College of medical directors (at SSF level) within 30 days after the introduction at the local sickness fund. If a file is incomplete, this of course will affect on the period needed to process the file. Since the handling period is one of the criteria that influence the overall funding of the administrative costs of the national sickness funds by the NIHDI, the sickness fund will 'suspend' the period for treatment of the application until the missing elements or documents have been provided by the applicant. In some cases, the medical administration of the national sickness fund will directly contact the prescribing doctor or the hospital or medical centre where the patient is treated in order to obtain the missing elements.

Once the administrative control is completed, the files are transferred to the medical director of the national sickness fund. For all national sickness funds, but one, the SSF files are handled by a single medical director. For one of the national sickness funds, there are two medical directors involved, treating separately the applications for the Dutch speaking members of the sickness fund and these of the French and German speaking members. These medical directors all are as well a member of the 'College of medical directors' at the SFF level. The practical consequence is that they mostly will have to decide (as a member of the College at the SSF level (cfr. Infra), on the applications they treat, and advice upon, at the level of the national sickness fund.

As will be illustrated further in this report, treatment by the 'College of medical directors' is one of the three possible decision-making processes at SSF level for applications for reimbursement.

The medical director will examine the file and, if adequate, add additional information on the case, taking into account the criteria to be met for reimbursement by the SSF. Additional information mostly refers to medical information on the rare disease the patient is suffering from, the indications, or on the treatment, the medical device or the drug prescribed by the patient's doctor.

The medical director from the national sickness fund formulates an advice (positive or negative) to the SSF on each separate dossier, using the SSF criteria on reimbursement. He is not bound by the advice from the medical doctor from the local sickness fund. The medical director will not transfer the application to the College medical directors if it concerns (art. 25 septies §2):

- Travel costs or costs of stay during a hospitalization in Belgium of a patient or the accompanying person;
- Some costs as room supplements, honorarium- and price supplements, co-payments and comfort costs The co-payments that were not taken into account for the calculation of the MAB ceiling, as well as the delivery margin and the safety margin for costly implants however are eligible for reimbursement to chronically ill children under 19 years old (see also above).
- Medical costs, costs of stay or travel costs for care abroad which the medical advisor did not consented to;
- Interventions provided more than 3 years before the application.

As at local level, no SSF applications are rejected at national level since the patient is the sole party to decide on the introduction of a demand for reimbursement. Even if the advice from the medical director of the sickness fund is negative, the application still will be transferred to the SSF.

Neither the patient nor the local sickness fund is informed on the advice issued by the medical director of the national sickness fund. They both will only receive the final decision that is taken at SSF level by the 'College medical directors' or by the SSF leading medical doctor (in case of delegation).

5.2.4 Process flow within the SSF

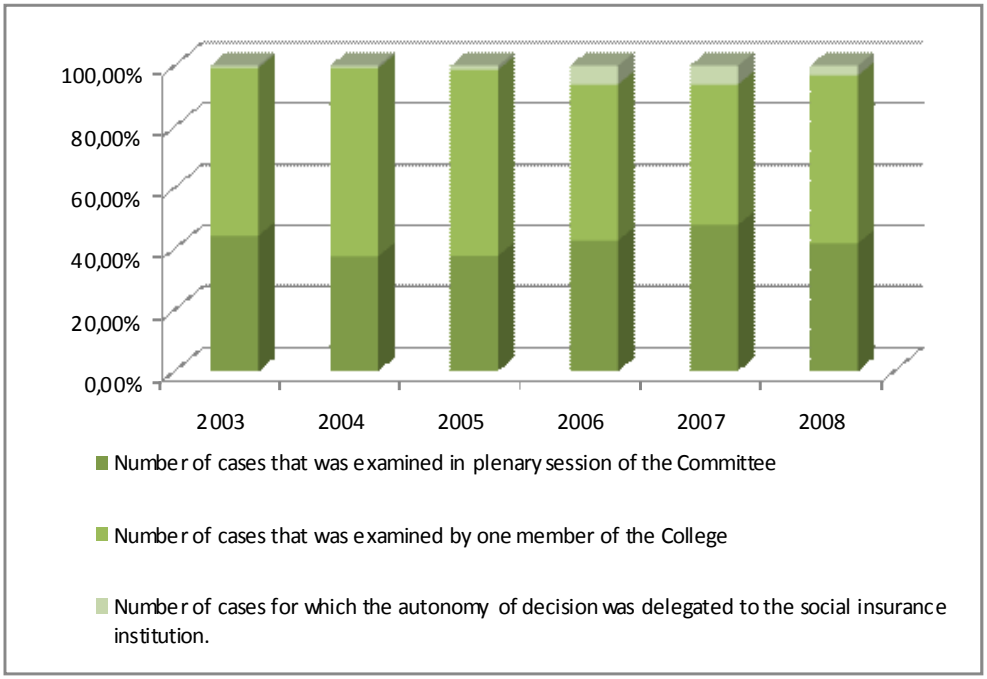
5.2.4.1 The decision-making processes on patients' applications

There are three different decision-making processes that can be used to decide on a patient's application:

- Patients' applications treated in the plenary session of the 'College of medical directors'.
- Delegation of decision by the 'College of medical directors' to one member of the college (actually to the SSF leading officer that is a medical doctor)
- Delegation of decision on patients' applications towards the local sickness funds

In the graph below (figure 6) the repartition between the 3 decision-making processes is shown.

Figure 6: Distribution of the three SSF decision-making processes

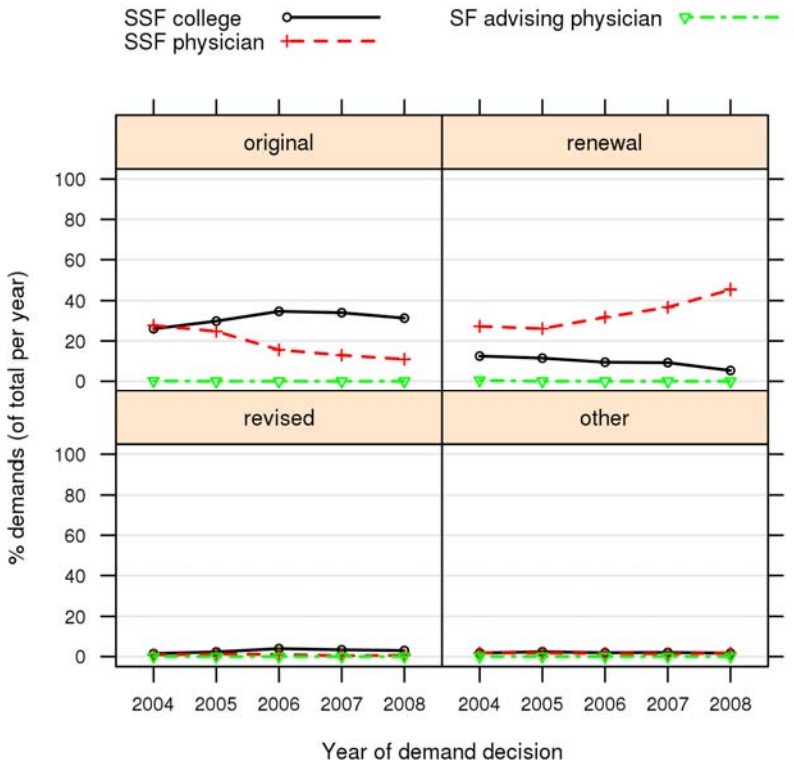


Source: Annual reports of the Special Solidarity Fund 2005, 2006, 2007, 2008

The data from the SSF sample suggest that the SSF College has remained the dominant decision organ for new applications and revision of applications, while requests to extend a previously introduced (and treated) application [16] were more and more delegated to one member or, to a lesser extent, to the sickness funds (see figure 7).

16 Requests for extension of a previously treated application are treated procedurally by the SSF as new applications: the same rules and formalities apply.

Figure 7: Proportion of applications in the SSF sample in function of decision organ and type of demand by year of decision (SF:sickness fund)



The law provides that the King can define conditions for the delegation of the applications to one or more physician of the College. The amount, the invoice and the payment of reimbursement are criteria that can be taken into account. The decisive competence can not exclusively be exercised by a physician of the sickness fund the patient is affiliated to. Today however there are no formal rules to orient the applications to the central (College) or decentralised decision process (delegation to the SSF leading officer (medical doctor). The SSF medical doctor decides on the routine applications by delegation of the College. Decisions on what types of cases are delegated are taken by the College. These decisions are mentioned in the minutes of the meetings of the College.

The policy of the SSF leading officer to whom the delegation is given, is that if there is any doubt whether the case meets the requirements of the delegation or if there is any doubt whether the application meets the criteria for reimbursement, the application will be submitted to the plenary session of the 'college of medical doctors'.

There is no formal control on the delegated decisions taken by the SSF leading officer. However, all negative decisions will always be contra signed by one member of the College.

Decisions that are delegated to the (local) sickness funds are limited. Currently all EB (Epydermolysis Bullosa) files are delegated to the sickness funds since 2001.[10] In the Royal Decree, the possibility was created to further extend the delegations towards the sickness funds. This could be done by the College of medical directors. Each trimester the sickness funds have to enter a 'financial overview 'of the delegated decisions. The SSF medical doctor has no clear view on these cases. The application files are not transferred to the SSF services. As a consequence a full control of these delegated decisions is not possible.

5.2.4.2 Administrative handling of application files

Once a demand for reimbursement is submitted to the services of the SSF, the application is subject to administrative control procedures.

Administrative staff SSF

The administrative staff of the SSF counts 3 collaborators and 1 person for the handling of mail representing 3,6 FTE. Two of the staff members treat the more difficult applications. The applications are divided between these two staff members according to the language of the patient requesting for reimbursement.

Registration and handling of SSF applications

The applications are registered into the SSF administrative database. At the same time they are separated in two groups: the applications to be decided by the 'College of medical directors' and the ones to be decided 'by delegation'. (one member of the 'College of medical directors' – in reality the medical doctor in charge of the SSF).

As previously explained, if the request for reimbursement is a prolongation of a medical treatment or cost that previously was reimbursed, the request for reimbursement will be decided upon by the SSF medical director (leading officer of the SSF) 'by delegation'.

The SSF administrative staff checks the files on completeness. If elements are missing, (for example the prescription of a treatment or the motivation of the medical doctor issuing the prescription, the 'declaration on honour', the invoices), the staff will contact the national sickness fund and ask for the missing documents or return the file for completion.

The administrative staff of the SSF prepares the file for decision by the 'College of medical directors' or by the member of the college (delegation procedure) They enter the data from the application file into the SSF database.

The information included in the database is described in table 1 in section 4.1.3.

At the same time a paper sheet is completed. This sheet contains all information initially registered in the database and is used to register all elements that are important for the decision to be taken on the request for reimbursement. The administrative staff examines the case and formulates a draft decision as well as a draft motivation based on the criteria as foreseen in the SSF regulation.

If a non-reimbursement decision is suggested, the administrative staff will mention the criteria upon which the suggested rejection is based.

Preparing a draft decision, the administrative staff of the SSF adds information to the file on previous decisions of similar cases taken by the College of medical directors. They document the case and add relevant information to the file (for example on the prevalence of the illness, the drug prescribed…).

The administrative services of the SSF keep a track of all decisions taken at SSF level. All decisions of the 'College of medical directors' and the decisions taken 'by delegation' on reimbursement are stored in a file (text file) by the administrative staff.

For each application for reimbursement the administrative staff also calculates the cost of an eventual reimbursement over a one year period, in order to check if the criterion "the treatment or the medical device is expensive" is met.

Suspension of handling of the application

If the application has missing documents or is not complete, the administrative staff will suggest suspending the period for handling of the application.

The decisions on suspension are always taken by the leading medical officer of the SSF. If there is a suspension, the application will be returned to the correspondent sickness fund (national level).

Formal reasons for suspending are:

- Prescription is not included
- No medical record
- No declaration 'on honour'

If an invoice of medical costs is missing, the application will not be suspended; a decision on the request for reimbursement will be taken but under the condition that the missing invoice is provided.

The number of cases that are suspended is limited. The sickness fund at national level will have to complete the missing 'formal' elements of the request for reimbursement and re-enter it to the SSF services. When arriving at the SSF, it will be treated as a new request and registered as such in the SSF database.

5.2.4.3 Medical handling

After the administrative handling, the patient's file is transferred to the leading officer (medical doctor) of the SSF. The application and the draft decision as well as the motivation are reviewed in the perspective of the SSF criteria that have to be met from a medical point of view. The leading officer of the SSF will further document the case from a medical point of view and if necessary collect additional information from medical specialists and experts on the subject to be examined.

Using the file as prepared by the administrative staff, the leading officer of the SSF will check if the medical criteria as foreseen in the regulation on the SSF reimbursement are met.

If the conclusion of the SSF leading officer is to suggest to grant reimbursement, the corresponding category of the criteria that are applicable considering the SSF regulation, are mentioned on the paper sheet in the file. (sheet that has been prepared by the administrative staff).

If the request for reimbursement does not meet the SSF criteria, all criteria that are not met are mentioned (marked) on the back of the paper sheet.

This paper sheet is an important document since all elements for taking a final decision are inventoried. This sheet also is the official document on which the final decision (delegated decision or decision from the 'College of medical directors) will be mentioned and signed.

When the decision is a delegated decision, taken by one member of the 'College of medical directors', positive decisions will be signed by the person taking the decision. At this moment delegation is only given to the leading officer (medical doctor) of the SSF.

Delegated decisions to refuse reimbursement always will be contra signed by another member of the 'College of medical directors'. The current practice is, that the medical director from the sickness fund to which the patient adhered to, will contra sign the decision.

5.2.4.4 *Decisions on reimbursement by the 'College of medical directors'*

Process

The files that are submitted to the plenary session of the 'College of medical directors', are fully prepared by the SSF services. This college is composed of the medical directors (or their representatives) from each of the sickness funds and of medical doctors of the NIHDI (included one representative of the Drug Reimbursement Commission).

A 'proposal for decision' as well as a motivation from the leading officer of the SSF is mentioned in each file. The agenda as well as all application files are sent in advance to all members of the College. There are weekly meetings of the College.

A draft agenda, some accompanying documents and the minutes of the previous session are send in advance by email.

The final agenda and the files from the patients are delivered to the members of the College and normally at least two days before the meeting of the College.

The meetings of the 'College of medical directors' are chaired by the medical director of the NIHDI. The leading officer of the SSF weekly meets the chairman of the 'College' to prepare the meeting. The agenda and the individual cases to be discussed on the next meeting are reviewed.

Meeting of the 'College of medical directors'

The agenda for the meeting of the 'college' is mostly well filled. Beside the SSF files, the 'college' has many other items to treat. From the interviews with the members of the College we understood the time for the SSF agenda is rather limited. In order to proceed rapidly, the chairman formulates a proposal for decision on each application that has to be treated. The members of the College are asked if they agree with the proposal. Every member of the 'college' can ask to discuss the case submitted for decision. Argumentation and additional information can be provided at the meetings. If there is no consensus, a final decision is taken by a majority of votes. Additional information regarding the price and/or the indication can also be asked to external experts. The law refers to authoritative scientific organisations, official institutions within the NIHDI, the FOD Volksgezondheid, Veiligheid van de Voedselketen en Leefmilieu/SPF Santé publique Sécurité de la Chaîne alimentaire et Environnement and the Belgian Healthcare Knowledge Centre. If no advice was given within two months starting form the demand for additional information, the College decides. The decisions are included in the minutes of the session of the College. These minutes are formally approved at the next meeting of the College.

5.2.4.5 *Handling period within the SSF*

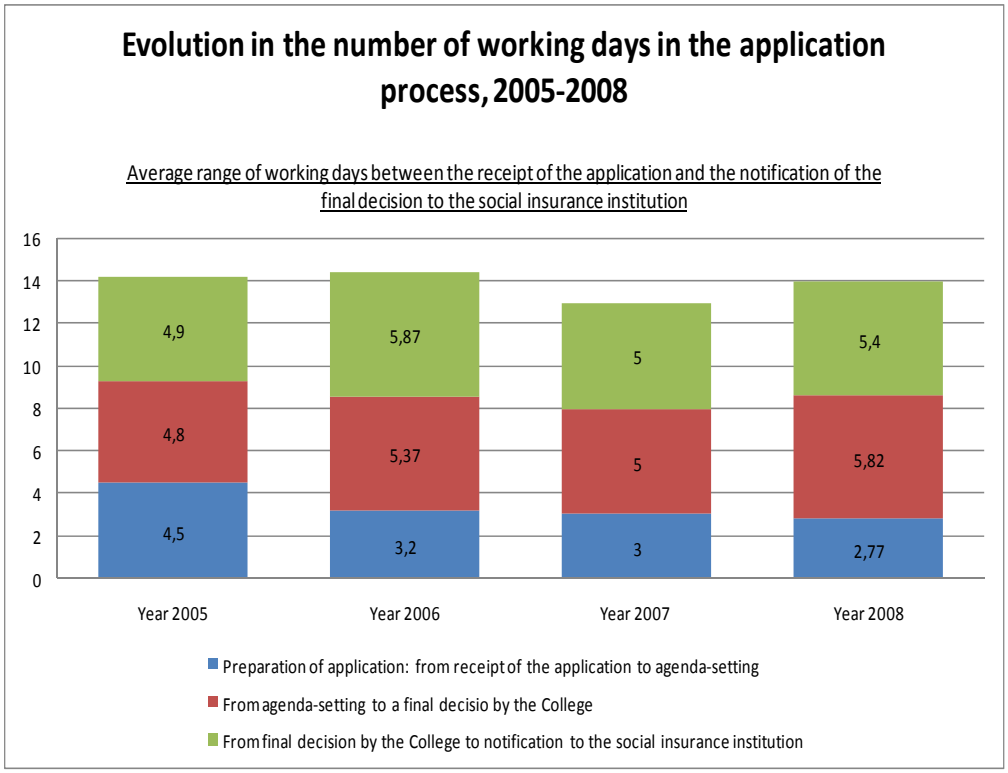
As shown in the table below (table 7), the average handling period within the SSF has been reduced over the years. In the SSF annual reports, it is clearly stipulated that the 2003 data are not comparable with the next years. In the graph below the results are visualized for the years 2005, 2006, 2007 and 2008 showing the total process time within the SSF evolved from 14 days to 12 days.

Table 7: Throughput time of SSF submissions within the SSF

Number of working days	2004	2005	2006	2007	2008
Average range of working days for the preparation of the application: period between the receipt of the application (by NIIS) and the inclusion of the demand on the agenda of the College	4,3	4,5	3,20	3	2,77
Average range of working days between the inclusion of the application on the agenda of the College and the final decision	3,89	4,8	5,37	5	5,82
Average range of working days for the notification of the decision of the College to the social insurance institution: period between the final decision and the notification (minutes of meeting, adoption of the minutes of meeting by the members...)	5,06	4,9	5,87	5	5,4
Average range of working days for the notification of the final decision to the social insured: period between final decision and the notification (minutes of meeting, adoption of the minutes of meeting by the members....)	4,95	4,9	5,87	5	5,3
Average range of working days between the receipt of the application by the NIIS and the notification of the final decision to the social insurance institution.	14,75	14,3	14,44	13	12,37

Source: Annual reports of the Special Solidarity Fund , 2004, 2005, 2006, 2007, 2008

Figure 8: Throughput time of SSF submissions within the SSF



Source: Annual reports of the Special Solidarity Fund 2005, 2006, 2007, 2008

The SSF sample allowed us to study the throughput time of applications in more detail. For the current analysis, throughput time is defined as the number of days between the reception of the application by the SSF and the date the notification letter to the insured was mailed. Also, no correction for suspended applications due to missing information, was needed as these were removed from the analysis dataset (5.57% of all available applications, see methodology section).

Overall, over 90% of the applications are completed within a month with half of them within two weeks (see figure 9). The rate of completion of accepted versus declined applications seems to be associated with the type of application (see figure 10). For about 40% of the original applications, accepted applications were treated in a shorter time compared to declined applications, while the opposite was true for about 5%. No difference was found for the remaining applications. For original applications, almost 90% were treated within one month.

For requests of renewal of previously treated applications, all eventually declined applications took a longer time to treat compared to accepted applications (see figure 10). Also, about 22% of the declined applications for renewal took over one month to complete compared to only about 6% of the accepted applications for renewal.

Figure 9: Proportion of completed SSF applications in the SSF sample in function of time between 2004 and 2008 (applications taking longer than 60 days are not shown)

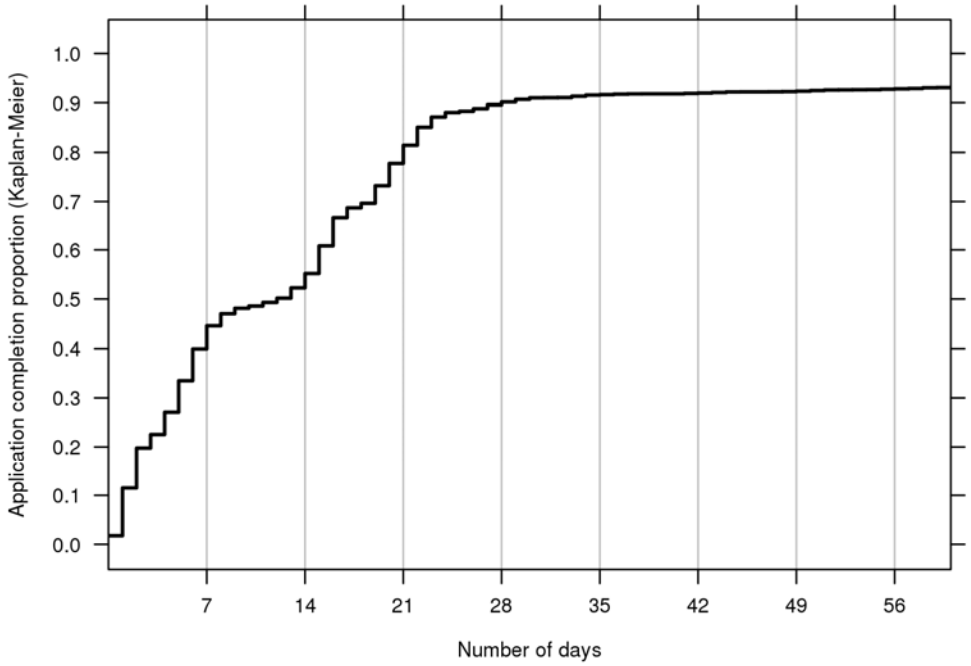
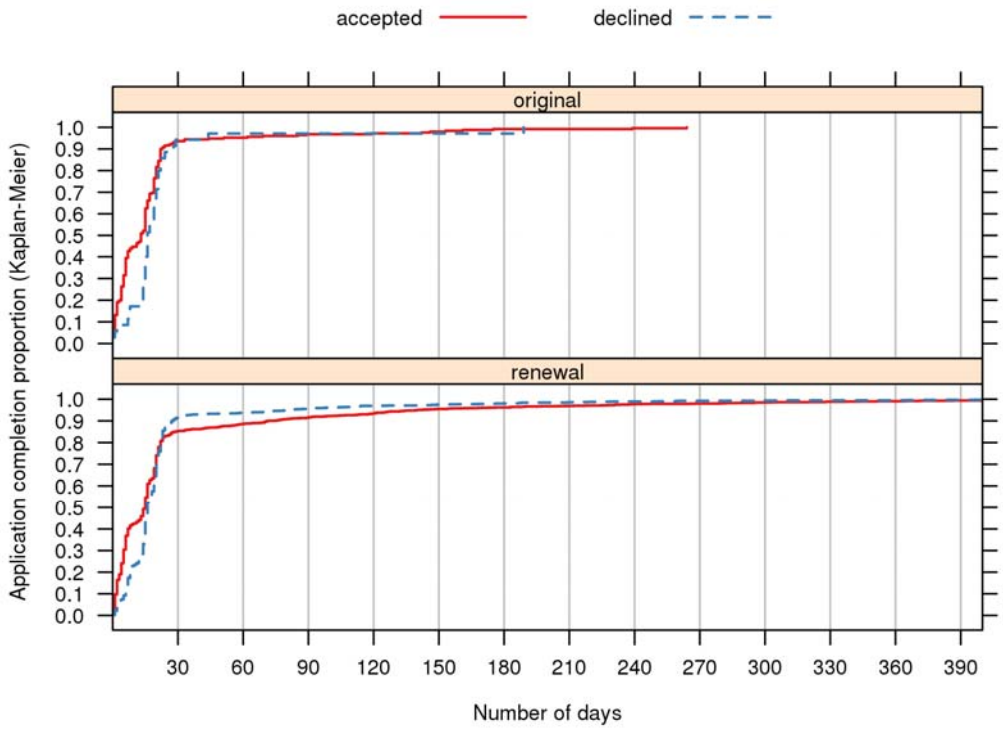
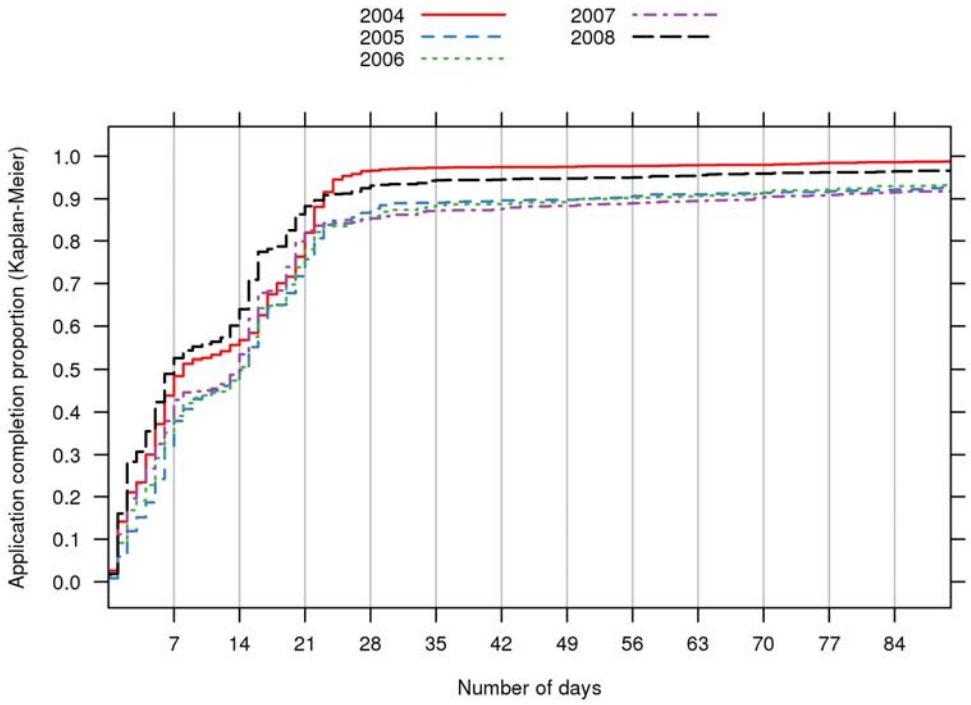


Figure 10: Proportion of completed SSF applications in the SSF sample by type of application and decision in function of time between 2004 and 2008 (applications taking longer than 1.1 year are not shown; applications of the type revised and other are not shown [7.2%])



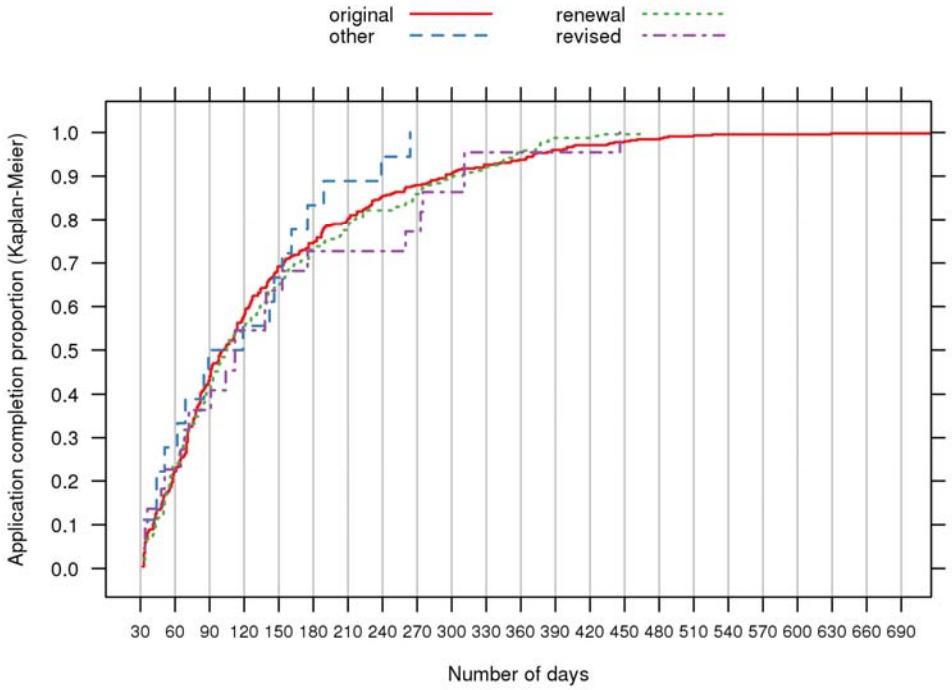
There does not seem to be a large difference in terms of the year the decision was taken on the throughput time (see figure 11). In 2004 and 2008, there was a smaller number of applications that took over a month to complete compared to the other years, but the large majority of applications were handled within a month irrespective of the year the decision was taken.

Figure 11: Proportion of completed SSF applications in the SSF sample by year in function of time between 2004 and 2008 (applications taking longer than 90 days are not shown)



As noted above, less than 10% of the applications has a throughput time of over a month. Of these applications, about half of them are handled within six months. The applications that take the longest tend to be new applications (see figure 12).

Figure 12: Proportion of completed SSF applications in the SSF sample by year in function of type between 2004 and 2008 (only applications taking longer than 30 days are shown)



5.2.5 Appeal procedure

If the patient disagrees with the decisions of the SSF, he can launch an appeal to the labour court (Art. 580, 2° Gerechtelijk Wetboek[17] en art. 167, 1° van de Wet betreffende de verplichte verzekering voor geneeskundige verzorging en uitkeringen, gecoördineerd op 14 juli 1994).[1817]

The content of the competence of the labour courts was for a long time under discussion. It was affirmed by jurisprudence and doctrine however that if the College of medical directors judges on the eligibility criteria, this is not a discretionary power.[22] Discretionary power implies freedom of judgement (when a criterion is vague and has to be interpreted) and policy freedom (freedom to decide, even if a situation complies with the criteria). The fact that the College can only strictly apply the legal criteria implies that in case the labour courts have full judicial power (on the reasonability of decision-making and on the content of the decision). With regard to the amount that will be allocated however, the college has discretionary powers. Consequently labour courts solely have the competence to judge the reasonability of decision-making, not the content of the decision (= the amount). This implies that a judge can annul the decision of the College on the allocated amount but not judge on the amount itself.

The numbers of registered cases that were introduced to the labour courts varies from 100 in 2004, 74 in 2005, 44 in 2006, 52 in 2007 to 23 in 2008. These numbers reflect the number of cases that were brought to court during those years. They can be related to decisions of the SSF from different years. In the SSF sample, the applications treated by the labour court amount to 144 decisions (1.75% of all applications and 2.56% of all accepted applications). Mostly decisions concerning diseases of the nervous or the circulatory system (see table 8); and implants and drugs are brought before the labour court (see table 9).

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17 For an overview of judgments of the labour courts, J. Diependaele, "Het Bijzonder Solidariteitsfonds, blijvende bron van controverses"[19], J. Put, "Het Bijzonder Solidariteitsfonds: interpretatie en beleid", noot onder Arbeidshof Gent 3 maart 2000[20], W. Bourry, "Het bijzonder solidariteitsfonds: een deur die nooit opengaat".[21]

Table 8: Applications before labour court in function of ICD-9-CM diagnosis group

ICD-9-CM diagnosis group		Percentage of all labour court applications	Percentage of all applications
320-389	Diseases of the Nervous System and Sense Organs	31,3%	24,8%
390-459	Diseases of the Circulatory System	16,7%	25,2%
140-239	Neoplasms	13,2%	8,6%
800-999	Injury and Poisoning	6,3%	3,2%
580-629	Diseases of the Genitourinary System	4,9%	4,9%
680-709	Diseases of the Skin and Subcutaneous Tissue	4,9%	2,4%
710-739	Diseases of the Musculoskeletal System and Connective Tissue	4,9%	4,6%
740-759	Congenital Anomalies	4,2%	4,0%
290-319	Mental Disorders	3,5%	0,9%
520-579	Diseases of the Digestive System	3,5%	4,7%
460-519	Diseases of the Respiratory System	2,8%	1,5%
240-279	Endocrine, Nutritional and Metabolic Diseases, and Immunity Disorders	1,4%	10,1%
780-799	Symptoms, Signs and Ill-defined Conditions	1,4%	1,8%
280-289	Diseases of the Blood and Blood-Forming Organs	0,7%	1,0%

Table 9: Applications before labour court in function of health care service

Health care service	Percentage of all labour court applications	Percentage of all applications
implants	27,1%	16,9%
drugs	25,7%	49,4%
instruments, devices, orthosis	16,0%	7,6%
innovations	15,3%	13,3%
health care abroad	9,0%	3,6%
ointments, drugs, bandages skin diseases	2,8%	1,0%
fee	2,1%	0,8%
special diet	2,1%	2,5%

The judgements of the labour court are not systematically monitored by the SSF and do not serve as guidelines for decisions on individual cases (personal communication medical directors). This is not so surprising since there's no continuity and uniformity in the interpretation of some of the eligibility criteria by the courts. The following examples illustrate the divergence of judgements.

- Expensive

There is no consensus between labor courts about the fact if the personal financial situation of the respective patient has to be taken into account. For instance, the labor court of Antwerp[23] stated that given the social basis inherent to the SSF, the assessment of the term "expensive" has to be related to the financial situation of the patient. The labor court of Gent[24] on the other hand judged that the notion "expensive" has to be set according to objective standards and in function of the treatment and the aim of treatment.

- Threatening the vital functions of the patient

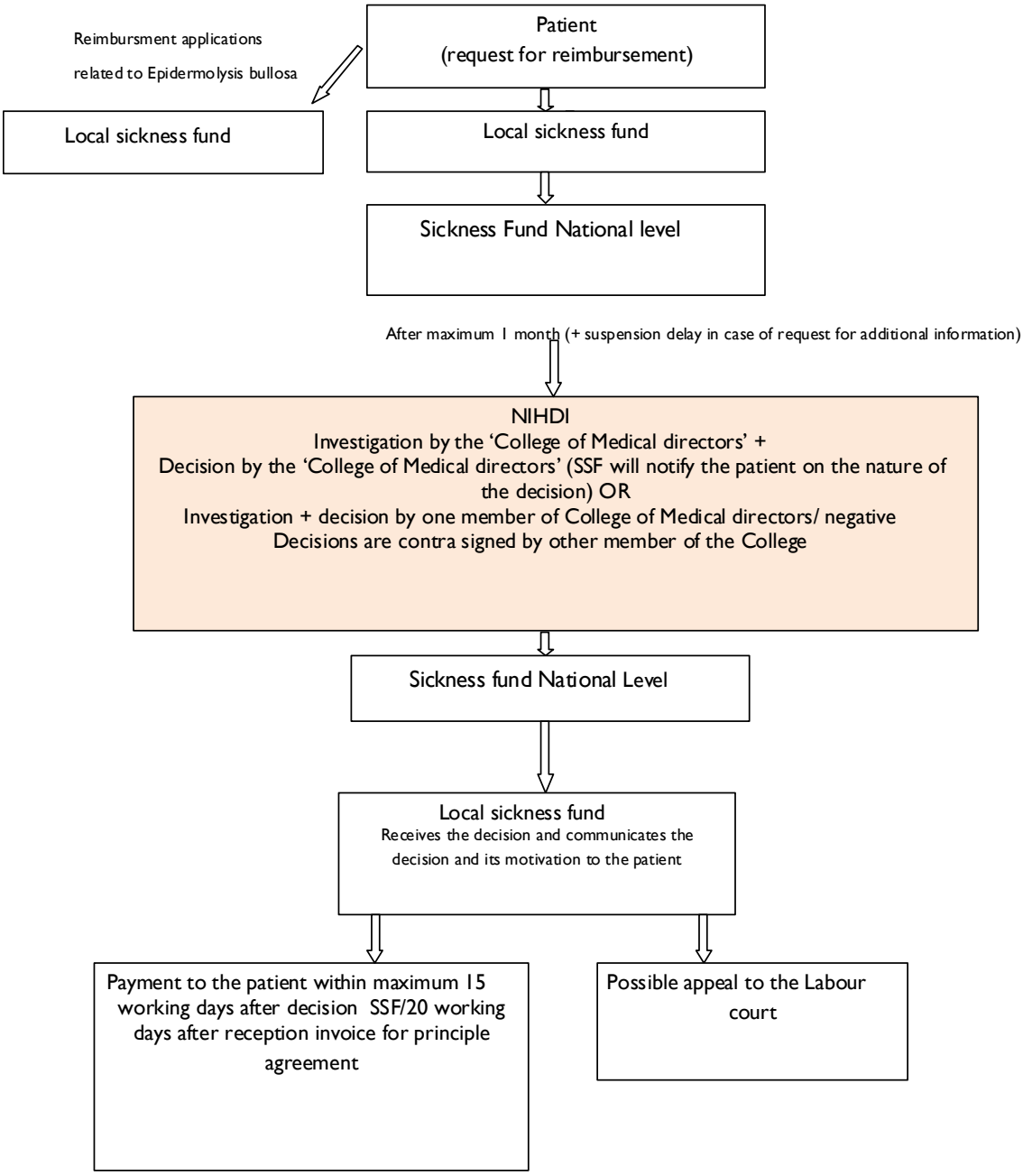
There is a tendency towards a broader interpretation of this criterion in Jurisprudence. According to the strict interpretation of the Labor Court in 1999[25], the criterion has to be understood as following: "necessary functions of the human body, such as conscience, respiration, the heart and blood circulation".[18] Broad interpretations refer to a primordial interest for living or functioning[27]. In that scope psychosocial diseases and quality of life can also be taken into account.[28-30] In the preparatory documents of the law of 2005 the Minister has explicated that a vital function is an essential function such as the sight, even if the disease the patient is suffering from is not life threatening.[31] The labor Court of Antwerp supported this vision in its decision of 10 May 2005 stating that the requirement of the life threatening character of the disease adds a criterion that is not provided in law.[32]

- Steps from application to reimbursement

The consecutive steps from submitting a request for reimbursement till the final payment of the amount approved by the SSF are summarized the figure below:

18 other court rulings supporting the strict interpretation: Arbh. Gent 10 mei 2002, vijfde kamer, A.R. nr. 2001/164, quoted by J. Diependaele [19]; Arbh. Gent (afd. Gent) 14 juni 2004[26]

Figure 3: Consecutive steps of an SSF submission



Keypoints: description of the functioning of the SSF

- **The SSF is operational since 1990. The actual regulatory framework is the law of 27 April 2005.**
- **The SSF reimburses costs of medical treatments that are not covered by the compulsory health care insurance system (or any other insurance) and that are related to rare indications, rare diseases, innovative treatment techniques, chronically ill children and medical treatment abroad.**
- **To be eligible for reimbursement by the SSF, the medical treatment has to be expensive, threatening the vital functions of the patient, have a proven scientific value and effectiveness and be prescribed by a specialized medical doctor. There may be no alternative that is reimbursed by the compulsory health care insurance system and the patient may not have other rights for reimbursement from other private or public insurance systems.**
- **In the SSF sample, we found that in particular for drugs and special diet, applications are often reimbursed at 60%, 75% or 85%. The majority of the applications originates from a limited number of prescribers or hospitals. Applications are in 90% of the time treated within a month following reception by the SSF. Treatments specified in eventually accepted applications are on average more expensive than rejected applications. Labour court appeals do not seem to be limited to specific diagnosis or treatments.**
- **The budget of the SSF varies substantially over the years. Expenses are often determined by changes that occur in the reimbursement of medical costs in the compulsory health care insurance system. Reimbursement of medication is by far the largest part of the SSF budget. It counts for more than 92% of all SSF expenses.**
- **Applications for reimbursement of medical costs by the SSF start from an individual request introduced by the patient at the local sickness fund level that transfer them to the national sickness funds. From there the application passes to the SSF.**
- **Applications are examined from an administrative point of view (check if all required documents are present) and a medical point of view (check if the application meets the medical criteria for reimbursement by the SSF).**
- **Decisions on SSF applications are taken by the College of medical directors. For certain types of applications the decision can be delegated to one member of the college or to the local sickness funds. Appeals are handled by the labour courts.**

6 EVALUATION OF THE FUNCTIONING OF THE SSF

A high level description of the perceived positive and negative elements regarding the functioning of the SSF and some suggestions is listed in the current chapter. It should be stressed that these statements represent the perception and the experiences of the interviewed stakeholders. An overview of the detailed answers on the following topics is included in annex 9.10., 9.11. and 9.12. Annex 9.13. reflects the statements of Pharma.be and the representatives of the pharmaceutical industry.

- Knowledge on the existence of the SSF
- Need for a safety net
- The SSF: meeting its objectives
- Alternatives for the SSF
- The intervention domains of the SSG
- The limitations of the SSF intervention domains
- Experiences with the SSF and knowledge of the SFF procedure
- Communication and information dissemination
- Knowledge and appreciation of the SSF criteria

6.1 THE MEMBERS OF THE 'COLLEGE OF MEDICAL DIRECTORS'

As mentioned above, the College of Medical directors is composed out of the medical directors of the sickness funds and some medical doctors from the NIHDI. They meet on a weekly base to decide upon the SSF applications that have been introduced to the SSF services.

6.1.1 Primary objectives of the SSF

According to the medical directors interviewed, the primary objective of the SFF is to act as a social safety net for patients who are confronted with high, non-reimbursable costs following an expensive medical treatment. The SSF reimburses the expenses of a very small target population for rare treatments that are not reimbursed under the compulsory health care insurance system.

Some respondents consider the SSF as a contemporary solution ('waiting room') for specific and complex situations (treatments, devices or drugs) for which there has not been arranged a framework for reimbursement yet. Diseases, indications or medical situations that cannot be categorized based on a first analysis, risk to fall as a residual under the SSF umbrella. Some members of the 'College' are slightly worried about this evolution and stress that it would be better to speed up the inclusion of a number of expenses for medical treatments or medicines in the compulsory health care insurance framework.

There is a general concern among the respondents about the SSF refunding expenses for medical treatment or medicines that could easily be covered by the compulsory sickness insurance system especially for drugs and medical devices. However, pharmaceutical companies are not always eager to introduce a request at the "Drug Reimbursement Commission" (Commissie Tegemoetkomingen Geneesmiddelen/Commission Remboursement des Médicaments), deciding on what drugs, products or treatments are refundable within the compulsory health care insurance system. Since the introduction of such a request is costly procedure, commercial or financial arguments could explain the identified hesitation especially among pharmaceutical companies to start up the process. Entering an application for reimbursement of a drug in the compulsory health system also includes a discussion on price setting.

Some respondents state that the SSF may not result in inequalities between patients with a disease – indication versus a rare disease indication. The rare character may not lead to the situation that costs that have to paid out of pocket by patients with a non-rare disease/indication are reimbursed to patients with a rare disease/indication.

6.1.2 Relevance of the SSF

All respondents are convinced that the activities of the SSF correspond to real, identified needs. The relevance of the SSF becomes clear in a small number of rare, extraordinary situations for which the compulsory insurance system is not providing a solution yet.

Some respondents warn for the danger that the SSF might be used by the pharmaceutical industry as a fund for studies during phase 2 and phase 3 in the development process of a medicine. Today, the share of drugs in the total reimbursements by the SSF is already extremely high.

6.1.3 Familiarity of the SSF

According to our respondents, the SSF is still insufficiently known among health practitioners and health providers. Target populations might not be entirely reached because of the relatively limited knowledge of the SSF. To their opinion reaching the target populations in a more effective way is considered as a true challenge for the coming years.

Some respondents support the idea to shift the reimbursement of costs for 'chronically ill children' to the compulsory health system. They do not see this group of patients as a specific target population for the SSF and would prefer to have a regular reimbursement for the costs the SSF is reimbursing now on a case to case basis. The costs and the medical acts can easily be integrated in the standard nomenclature used in the compulsory system.

6.1.4 Procedure

6.1.4.1 Local Sickness Fund level

According to the interviewees, the local advising doctor should play a more active role during the first step of the procedure and examine eligibility to reimbursement more in detail instead of focusing on the administrative' aspects of the request.

6.1.4.2 SSF level

The involvement of the 'medical director' of the sickness fund at the SSF level in the SSF files is judged as relatively low due to a lack of time.

The preparation of the College meetings by its members is mostly limited to their individual files (belonging to their health insurance fund). Files of other members are available only two to one day before the meeting of the College, which is considered as a very short notice for preparation. This observation is corroborated indirectly by the SSF sample data. Of all applications, 95.5% is scheduled on a SSF College meeting within 7 days of reception. Applications delegated to the sickness fund are all scheduled within 5 days of reception.

The members of the College agree the leading officer is consequent in the policy to submit all cases to the plenary session of the College where there could be any doubt whether the application meets the criteria for reimbursement.

The fact there is no formal and systematic control on the decisions taken 'by delegation' is not judged as a problem. The contra signature of one other member of the College in the case of a refusal of reimbursement is seen as a sufficient control mechanism.

Based on the feedback of the members of the College, the reporting and follow up of applications, delegated to the SFF medical doctor could still be improved and be more systematically.

6.1.4.3 Appeal procedure

According to some of the interviewees the labour courts do not have sufficient expertise in assessing the criteria and the conditions for decision-making.

6.1.5 The criteria for decision-making

The decision-making criteria are not elaborated in depth. Different views exist between the members concerning the decision-making process. Some consider each case as an individual one (case-by-case handling), while others are striving for more uniformity in the decision-making process.

There is consensus between the interviewees that the legal criteria and requirements for eligibility are open for interpretation. Particularly, "expensive treatment", "rare disease", "experimental phase" and "vital functions" are terms that might cause confusion and discussion. Some respondents consider the broadness and vagueness of the criteria as an obstacle for coherent decision-making. However, most of them welcome the possibility of interpretation, given the role of the SSF as an additional safety net. The rareness of a disease is regarded in perspective to the orphaned criterion of a prevalence of 1/2000.

The 'College of medical directors' translates the criteria as defined by the law during the decision-making process in a pragmatic way. For the criteria "expensive" internal criteria per episode and per year are handled.

Whether the personal financial situation of the patient should be an element to be taken into account, when deciding on reimbursement (or portion of reimbursement) by the SSF, is an issue for some respondents. Today this is not a criterion applicable to the SSF reimbursement system

6.2 THE SOCIAL SERVICES

6.2.1 Brief presentation of the interviewed social services

In all the hospitals included in the sample, the social service is a central hospital department. Individual social workers are in all cases directly linked to one or more hospital wards and medical services. As a consequence they have a good knowledge of specific pathologies and interact with a limited number of permanent medical doctors. In university hospitals, characterized by a frequent turnover of medical doctors in training, social workers interact with more contact persons during a shorter time period.

The number of staff working in the social services varies between 3.5 FTE and 60 FTE. The range of tasks to perform is very extensive and diverse. It includes social and moral support, monitoring of complaints, handling payment difficulties of hospital invoices, completion of administrative formalities for example with the sickness funds, the organization of practical assistance related to the dismissal of the patient such as home care. For these tasks the initiators asking for support are patients, family, nurses or the medical doctors.

Providing support for and monitoring of SSF (special solidarity fund) files represents only a tiny fraction of the social workers' workload. The initiator asking for support from the social service in SSF cases is almost always the patients' treating medical doctor.

During our interviews no substantial differences in the answers were identified according to the different categories we distinguished (university versus non university hospital/French versus Flemish hospital/big versus small SSF applicant).

6.2.2 Positive elements regarding the existence and functioning of the SSF

- All respondents indicated that the existence of the SSF provides an answer to non regulated topics in the compulsory health care insurance system. The SSF is a social safety net, perceived as the ultimate "emergency solution" for some very specific cases.
- The SSF can detect and list non-regulated cases in the obligatory health care insurance system, provide information to the competent authorities on these cases and formulate suggestions on what cases should be included in the compulsory health care insurance coverage. This is seen as a "policy" task of the SSF.

6.2.3 Negative elements on the functioning of the SSF/suggestions for improvement

- The duration of the procedure (initiation to decision) is judged by nine of the twelve respondents as very problematic. The bottleneck is seen at the level of the sickness funds since an application, once it is send to the SSF, is quickly decided upon at SSF level. Especially for patients awaiting a decision of the SSF before starting treatment, important human dilemmas arise. An emergency procedure directly to the SSF is suggested as a possible solution.
- The procedure is defined as heavy (much paperwork) mainly for the doctors. For the social services the workload is usually seen as high but acceptable. Avoiding duplication of documents to be submitted, acceptance of "post factum" provision of the hospital's invoice document, acceptance of electronic signature would reduce the current administrative burden.
- The added value of the intermediate levels (sickness funds: local and national level) is unclear for the majority of the respondents and enlarges the throughput time of SSF applications. Submission of files directly to the SSF is suggested as alternative.
- The SSF is not very well known. This is a reality for employees of social services, medical doctors and smaller local sickness funds. An active approach from the SSF (yearly presentation on the functioning of the SSF and recent changes, more accurate information on criteria and decisions) would improve transparency and visibility of the SSF.
- "Open communication" by the SSF is judged non-existent. The internal functioning of the SSF (procedure) is unclear for the respondents. The lack of a clear motivation why cases are approved or denied (inclusion criteria/exclusion criteria) and why only a limited percentage of financial costs is reimbursed, is one of the reasons why lack of transparency is mentioned as a bottleneck for the SSF. Direct contact with the SSF is not allowed and consequently does not take place. For complex and serious cases, direct contact would be highly appreciated. It would increase the accessibility to the SSF and improve the image of the SSF, which is now perceived as a very isolated department/ non transparent institution.
- The administrative focus of the SSF sometimes overshadows the medical assessment of a file. Two SSF files were rejected as the patients died in the meantime and no "declaration on honour" could be added to the file.
- If the SSF intends to reject an application, it has to be motivated from a medical point of view. The prescribing and treating medical doctor should be able to argument the patients' case.

- The name "special solidarity fund" is confusing and creates the expectation that the financial situation of the patient is used as criterion, influencing the final decision of the SSF and the amount reimbursed. Several respondents indicate that the financial situation of the patients has to play a role in the assessment of a SSF file. One respondent would use this criterion as an eligibility criterion.
- The requirements asked by the sickness funds for SSF files differ regarding the required degree of detail. More uniformity in required documents could simplify the administrative structure of a file.

6.3 PATIENT ORGANISATIONS

6.3.1 Overview of the interviewed patient organisations

Table 10: Overview of interviewed patient organisations

	Number of interviewees
DEBRA (Dystrophic Epidermolysis Bullosa Research Organization)	2
PH Belgium (Pulmonary hypertension Belgium)	1
BOKS (Belgian organization for adults and children with metabolic disorders)	2
NEMA (Flemish patient organization for neuromuscular diseases)	1
Radiorg.be (Belgian patient organization for rare diseases)	1

A brief presentation of the interviewed patient organizations and their activities can be found in annex 9.11.

6.3.2 Positive elements regarding the existence and functioning of the SSF

- The main positive elements raised by the patient organizations are broadly the same as the ones perceived by the social services.
- All respondents indicated that the existence of the SSF provides an answer to non regulated topics in the compulsory health care insurance system. The SSF is a social safety net, perceived as the ultimate "emergency solution" for some very specific cases.
- Patient organizations are putting more the emphasis on the fact "a SSF system" needs to exist as it is crucial for many of their members affected by rare diseases. It is of less relevance for them by whom (what institution), under which system (compulsory health care insurance system or a separate initiative) or using which kind of procedure (decentralised or centralised) the reimbursement system is organized.

6.3.3 Negative elements on the functioning of the SSF / suggestions for improvement

- Patients are worried that the SSF is an emergency solution. The real issue is to have a solid and sustainable reimbursement system that guarantees equal and equitable treatment of patients having serious rare diseases, now and in the future. Why should we exclude a patient from receiving an essential but expensive treatment? Each patient has the right to receive the 'necessary'/'optimal' treatment even when financial costs are high. Accessibility from a financial viewpoint needs to be guaranteed, through the solidarity principle.
- The duration of the procedure (initiation to decision) is judged as too long although respondents noticed a decrease of the throughput time over the last years.
- The procedure is defined as heavy and complicated (much paperwork) mainly for the doctors.

- The added value of the intermediate levels in the centralized procedure (mainly the local sickness funds) is not clear for the majority of the respondents and unnecessarily enlarges the throughput time of SSF applications.
- Lack of knowledge within the local sickness funds should be eliminated from a patient's perspective. Centralization of knowledge and the development of single point of contacts would mean of world of difference for the patients.
- The SSF is not very well known. This is a reality for the patient organizations and their members but also for medical doctors and local sickness funds. A proactive approach from the SSF (ask the patient organizations to act as information disseminators towards their members) or giving a lecture on the SSF activities on an annual basis to the patient organizations would improve the visibility of the SSF.
- The SSF is judged as a very isolated department, mainly because direct contact with the SSF is not allowed and, as a consequence, does not take place. For complex and serious cases, patient organizations find it weird that the whole SSF evaluation is purely based on paper documents. A patient is never asked to show up or to explain / clarify his situation. More openness would be highly appreciated. It would increase the accessibility to the SSF and improve the image of the SSF.
- Most of the criteria, used by the SSF are not clear and not well known or very susceptible to interpretation.
- The administrative focus of the SSF sometimes overshadows the medical assessment.
- The name "special solidarity fund" creates the expectation that the financial situation of the patient is used as criterion, influencing the final decision of the SSF and the amount reimbursed. This is currently not the case and 3 of the 4 patient organizations do not want this to happen. For them, all patients confronted with a rare disease have the right to equal reimbursement regardless of their financial situation.
- All patient organizations support the view of clustering patients with rare diseases and treat them in a limited number of hospitals or reference centres. This will improve the quality of the medical care, the development of knowledge centres and allow structural support to be strengthened. Now patients still have to search for existing initiatives and accurate care provision and support systems including the SSF themselves. In an optimal situation this should not be their primarily focus since facing the diagnosis of a rare disease they have other primary concerns to handle.
- The clustering of patients in a limited number of reference centres would also ease the contacts and foster information exchange with the SSF.

6.4 THE MEDICAL SPECIALISTS

6.4.1 Brief presentation of the interviewed physicians

For the selection criteria used to obtain the sample, we refer to the methodological part of this report. The thirteen doctors interviewed all are medical specialists. The medical disciplines they exercise are: anaesthetics (1), paediatrics (5), cardiology (1), neurology (4), pneumology (1) and gastroenterology (1). Sub-specialisations are: pain therapy (1), metabolic diseases (2), oncology and haematology (2), immunology (1), neuropaediatrics(2) , neuromuscular diseases (1), paediatric neurologist (1), pulmonary hypertension (2). All medical doctors are working in hospitals and treat their patients in the hospital setting (ambulatory care and inpatient care).

The sample is composed of five physicians who predominantly treat adults, four mostly children and four both patient categories.

All physicians interviewed treat patients for which SSF applications have been submitted in the past 5 years. They work in larger medical teams and most of them regularly introduce new demands for SSF intervention. One physician did not have recent SSF applications (not over the past two years) since the medical device he prescribed was integrated into the compulsory health care insurance system.

From the answers provided during the interviews we could not determine major different patterns in views to be linked to the selection criteria used for the sample of interviewees. There are no important differences in views that can be related to the size (large or small) or the type of hospitals (university or non-university hospitals) the medical doctors belong to, or the number of SSF applications they introduced.

6.4.2 Positive elements regarding the existence and functioning of the SSF

- All respondents see the SSF as the only actual way to assure patients suffering from serious diseases financial compensation for their, mostly huge medical costs. It is a necessary additional system to the compulsory health care insurance system. All respondents agree it is not possible to have every situation regulated and taken care off in the regular system. It will always be necessary to have a complementary system. It should be limited to those situations that cannot at all be regulated within the compulsory health care insurance system.
- The SSF gives the opportunity to prescribe new drugs, new medical techniques and devices that are not yet registered in Belgium. However price setting is badly taken care of, the fact these drugs can be prescribed and can be reimbursed is very valuable.

6.4.3 Negative elements on the functioning of the SSF / suggestions for improvement

- The SSF as a complementary system to the compulsory health care insurance system has to be as restrained as possible. Treatments, costs of drugs and medical devices that are prescribed or used regularly, have to be transferred to the general system. For the SSF this transfer is not always done in due time. Some drugs stay in the SSF system for much too long.
- The SSF has a role as a waiting room for new treatments, new drugs and new medical devices. Real assessment of these new techniques is not really undertaken.
- The difference between orphan drugs and drugs reimbursed under the SSF system is not always clear. Once a drug is recognized as an orphan drug, the SSF does no longer intervene even for indications that are not included in the acceptance from the drug as an orphan drug. This is not acceptable and ultimately leaves the patient in a no-man's-land.
- The procedures are too long and too complicated to provide an adequate and quick access to treatments that are medically urgent. The throughput time is not acceptable. Throughput time has to be limited and an emergency procedure for immediate decision has to complement the current procedure.
- The intermediate steps of the local sickness fund and the sickness fund at federal level is unnecessary and has no real added value. SSF applications have to be entered at the SSF directly eliminating all unnecessary steps.
- The criteria used by the SSF for deciding on reimbursement are not clear or specific enough. They all should be public.
- Information on decisions (principles) taken by the SSF should be public transparent and accessible.

- The use of new drugs as well as prescription of drugs for other indications then those registered, must be easier and integrated in the compulsory health care insurance system. Moreover there is a lack of clear motivation of the SSF decisions. Decisions have to be motivated from a medical point of view and not from a administrative one (as they are now).
- The outcome of an SSF application is not predictable. Several respondents (5 directly and 4 indirectly) said they obtained different decisions on similar cases.
- Decisions on SSF applications have to be taken by peers. One cannot expect the medical directors to have the knowledge and the expertise on such complicated medical cases and pathologies. Suggestions are: advice from independent experts; second opinion, post factum review of the medical files by expert, post factum assessment of the effectiveness of the treatment.
- The SSF has to have more confidence in the medical doctors that prescribe treatments for rare diseases or patients with very special specific medical conditions. Their expertise must be valued. One should start from an acceptance of the treatments they prescribe, not a refusal.
- When a negative decision (actual procedure) is envisaged, the respondents expect to be contacted in advance as to be able to add information or clarify the case.
- The SSF is totally unknown to the medical profession. Only one respondent said he (once) had a personal contact at SSF level. The SSF is a black box, hidden in the woods.
- The procedure for appealing a decision taken by the SSF is not adequate, an internal procedure has to be foreseen.

6.5 THE PHARMACEUTICAL INDUSTRY

To have the opinion of the pharmaceutical industry on the functioning of the SSF, we contacted the umbrella Belgian organisation Pharma.be. The perceptions of Pharma.be can be found in annex 9.13. Furthermore, representatives of four pharmaceutical companies, indicated by Pharma.be as the most familiar with the SSF expressed their opinion. The overall results can be found in annex 9.13.

6.5.1 Positive elements on the functioning of the SSF

- The SSF is seen as a system that provides solutions to patients for high medical expenses that are not covered by the compulsory health care insurance system.

6.5.2 Negative elements on the functioning of the SSF / suggestions for improvement

- According to Pharma.be, there is a need to have a specific system for "early access" to new drugs that have already obtained the EMEA market authorisation but where there is no decision yet at Belgian level for reimbursement of the drug. If market authorisation has been obtained, the pharmaceutical industry finds it is the responsibility of the compulsory health care insurance system to cover the costs.
- According to the interrogated pharmaceutical companies, the time that is needed for acceptance of new drugs (after EMEA registration) into the Belgian compulsory health care insurance system has to be shortened.

- Compassionate use and medical need programs are not seen as a structural solution for access to medication that is not accepted yet in Belgium but that (in the case of medical need programs) obtained the EMEA market authorisation. For smaller pharmaceutical companies it is not obvious to bear the cost of compassionate use. Compassionate use is not seen as a structural solution for off label use of medication.
- The interviewed pharmaceutical companies state that for drugs that are used for rare indications, it is not obvious to ask for EMEA market authorisation. The cost for the studies is too high in perspective of the number of cases concerned. In some cases scientific studies just cannot be performed since the patient groups are too small.
- Two alternative systems are suggested by the pharmaceutical companies. On the one hand, a risk sharing system where the public health care insurance system will only fund the costs of the medication if the patient reacts favourably to the treatment can be envisaged. On the other hand stopping rules where reimbursement stops when a patient does not respond to the treatment could be an alternative.
- Transparency of the criteria used by the SSF is perceived as poor.
- On the procedural level, the time to get a decision from the SSF is judged as unacceptably long, The internal procedures of the SSF as well as the preliminary steps at sickness fund level are perceived as not clear.
- The administrative burden for the prescribing medical doctor is judged as too high leading to cases where no application is introduced although theoretically the SSF could intervene.
- According to the pharmaceutical companies, decisions at the SSF are taken without having the expertise that is needed to do it adequately. The advice of the NIHDI Drug Reimbursement Commission is no alternative.
- The pharmaceutical companies are not at all involved in the SSF procedures. They have no knowledge or control at all on the introduction of an SSF application.
- The administrative follow up of the invoices for the medication by the hospitals is judged as very time consuming. In some cases the total amount of these invoices that are waiting for payment can be very important.
- The pharmaceutical companies ask to have a more transparent procedure where the SSF would keep them informed on the decision and the payment procedure. Such notification can be installed in full respect of the patients' privacy by making it completely anonymous.

7 EXPLORATION OF (COMPARABLE) SAFETY NETS IN A SELECTION OF FOREIGN COUNTRIES

The initial aim of the international approach was to find comparable safety nets for costs of treatment of rare diseases or rare indications in a selection of foreign countries or to see whether these are included in the universal cover. The countries that were selected are France, the Netherlands and Spain being countries with comparable health care systems, comparable living standards and having a geographically proximity to Belgium.

In none of these countries comparable safety nets as the SSF exist. In this chapter we give an overall view on the organisation of the health care system of those countries and describe how cost of treatments of rare diseases and rare indications are covered and how access to new drugs or innovative medical techniques is handled. A list of cases of costs that are reimbursed by the SSF has been used to gain information on how these cases were dealt with in these countries.

7.1 FRANCE

Background information on the French healthcare system and the organisation of healthcare provision can be found in annex 9.14.

7.1.1 The reimbursements of medicines

In order to grant new medicines access to the French healthcare market, a benefit/risk assessment by the European Medicines Agency (EMEA) and French Agency AFSSAPS (Agence français de la Sécurite Sanitaire des Produits de Santé) is necessary. Based on this assessment, medicines are granted Marketing Authorization (MA) for France.

Following, requests by manufacturers regarding the adoption of medicines on the list of reimbursable drugs are evaluated by the so-called Transparency Commission (*Commission de Transparence*) within the Haute Autorité de Santé (HAS). The assessment of the medicine is based on two criteria*:* the therapeutic value and the effectiveness of the medicine *(Service Medical Rendu, SMR)* which leads to a categorisation of the drug according 4 levels (important, modest, weak and insufficient), and the therapeutically added value of the medicine in comparison with the current situation (*l'Amélioration du Service Médical Rendu, ASMR)*. The ASMR incorporates five levels, going from 1 (great improvement) to 5 (does not do any better than the current standard).

The labelling by the Transparency Commission is important for the manufacturer, as it will undoubtedly influence the public price that will be negotiated with the Economic Committee of Health Products (Comité Economique des Produits de Santé, CEPS). The CEPS also negotiates on the volume of medicines. Based on the agreement that has been made between the manufacturer and CEPS, the Minister of Health and Social Security decides on the inscription of the medicine on the list of reimbursable drugs. The level of co-payment is defined by the National Health Insurance Fund (Caisse Assurance Maladie).

Drugs, such as orphan drugs, can be prescribed without having first received a Marketing Authorisation through:

- Clinical trial procedures, if no other, proper alternative is available;
- Authorisation for temporary usage, granted by the AFSSAPS (l'Agence Française de Sécurité Sanitaire des Produits de Santé), better known as the ATU procedure (see next section);
- Hospital preparations, if no, other proper alternative is available.

7.1.1.1 Authorisation for temporary use in France

The French ATU system allows early access to new promising drugs that are not covered by a MA in France. However, an ATU may concern medicinal products that already have a MA abroad or drugs under development. In most cases, however, an application procedure is pending at EMEA. The ATU procedure does not apply to the use of a medicinal product having a MA in France for an indication other than that envisaged in its MA : This off label use is the unique responsibility of the prescriber.

The following general conditions have to be fulfilled:

1. the drugs are used for the treatment of serious or rare diseases;
2. there is no other treatment available;
3. cost-benefit analysis of the treatment was conducted, with a positive result.

The drug which has been authorised for temporary usage needs to be used only for therapeutic purposes and is not expected to hinder any further clinical trials.

There are two types of temporary use authorisation:

- nominated ATUs, provided for a single patient not taking part in a clinical trial, under the responsibility of the prescribing doctor;
- and cohort ATUs for groups of patients, established at the request of the holder of distribution rights.

A cohort ATU must be accompanied by a simultaneous demand for a MA or an intention to introduce a MA (marketing authorization) dossier in the near future. Moreover a cohort ATU is subordinated to the implementation of a protocol for therapeutic use and information collection, established by AFSSAPS in collaboration with the manufacturer. AFSSAPS may consider it as necessary that such a protocol is also set up for certain drugs products made available within the framework of nominative ATU.

The use of medicinal products subject to ATU cannot replace a clinical trial and the aim is not one of investigation. The decision for ATU must not slow down the implementation or the continuation of clinical trials, alone intended to determine precise and essential elements concerning the benefit/risk ratio of a medicinal product. Indeed, only clinical trials make it possible to collect reliable data, in particular in terms of efficacy, safety of use, medicinal product interactions and therapeutic strategies, while authorizing the access to medicinal products without MA. Making medicinal products available according to the ATU procedure or for clinical trials depends in particular on the level on information available on the medicinal product in question. Generally, in the early stages of the development of the medicinal product, clinical trials must be favoured.

Evaluation

NOMINATIVE ATU

The evaluation criteria concern the pharmaceutical quality (viral safety if applicable) of the drug, its safety and efficacy in the indication claimed in the ATU and the absence of any therapeutic alternative.

Each named ATU application is studied by the AFSSAPS, assisted by experts. There are 5 evaluators and 1 chief of the ATU unit. Three of the members are pharmacists, the others are physicians. Given the large number of requests (+/- 20.000/year) the less complicated cases are handled by one of the members of the unit. It mostly concerns cases that are similar to earlier cases and/or renewals. More complicated problems are dealt with collectively. For very rare diseases an appeal is made to external experts.

The agency notably relies on a dossier on the medicinal product supplied by the holder of distribution rights at its request and, if necessary, by the ATU applicant and including, in particular:

- a copy of the authorisation obtained abroad, if applicable;

- any available information relative to the pharmaceutical quality, efficacy and safety (bibliography, investigator's brochure, etc.);
- a list of ongoing or scheduled clinical trials in France.

Possible decisions are:

- Granted: the following information is mentioned:
  - name of the medicinal product;
  - contact details of the prescribing physician;
  - patient's initials;
  - treatment duration;
  - contact details of the hospital pharmacist. The ATU is sent by fax then by post to the hospital pharmacist, who informs the prescribing physician.
- Rejected, for the following reasons notably:
  - existence of a therapeutic alternative with a MA and available on the market
  - and/or absence of convincing data suggestive of a real benefit for the patient
  - and/or use requested for investigative purposes.

The rejection is sent by fax to the pharmacist, who informs the prescribing physician, and by recorded delivery mail with acknowledgment of receipt to the prescribing physician and the pharmacist. A reapplication may be made to the Director General of the AFSSAPS and/or the matter may be referred to the relevant administrative court within a period of 2 months from notification of the decision. Nominative ATU is granted for a duration of maximally one year and can possibly be renewed.

COHORT ATU

For cohort ATU, the efficacy and the safety of use of medicinal products are strongly presumed, taking into account the clinical trial results performed within the objective of MA application, and that this application has been introduced or that the applicant commits himself to introduce it in the near future.

The application file includes:

- the reasons for the application;
- a commitment to introduce an application for MA and the expected date.
- an administrative dossier including:
  - if relevant, a copy of the application for MA project of ATU summary of product characteristics (SPC), patient information leaflet and labelling;
  - project of protocol for therapeutic use and information collection;
  - the titles and objectives of the ongoing clinical trials with their progress reports and the trials planned for the same disease in France or abroad;
  - the identity of the principal investigator(s) in France and the name of the research centre(s) concerned in France,
- A medicinal product dossier: The file contains all the pharmaceutical and pharmaco-toxico-clinical data available at the moment of the application (even if the studies are ongoing).

This protocol is drawn up by the manufacturer of the medicinal product concerned in close collaboration with the ATU unit at AFSSAPS. The aim of this protocol is:

- to provide prescribing physicians with any relevant information about the medicinal product and its use,
- to organise patients monitoring,

- and to collect information relative to the actual use of the medicinal product during the ATU and pharmacovigilance with the view of drawing up a periodic ATU report which is intended to be regularly transmitted to AFSSAPS.

Each cohort ATU application is assessed by the Marketing Authorisation Committee of AFSSAPS[19]. In particular, assessment deals with the pharmaceutical quality, safety and efficacy of the medicinal product for the indication claimed, the draft protocol for therapeutic use and information collection, the draft summary of product characteristics, the draft patient information leaflet and labelling, the prescribing and supplying conditions as well as the absence of therapeutic alternative available on the French market.

A cohort ATU is granted for a fixed indication which must be respected. To the authorisation are attached the summary of product characteristics, the patient information leaflet, the labelling as well as the protocol for therapeutic use and information collection. AFSSAPS also notifies the frequency of periodic ATU. Cohort ATU is mostly granted for a duration of one year and can possibly be renewed.

The response times for ATU applications depends, first of all, on the therapeutic emergency and, secondly, on the level of knowledge about the drug at the AFSSAPS. When the medicinal product has already been evaluated by the AFSSAPS, the decision is generally being made within 24-48 hours. When the medicinal product has never been evaluated, the response time depends on the duration to compile the dossier and to complete the assessment.

Periodic ATU reports are sent to AFSSAPS and, if applicable, to the regional pharmacovigilance centers in charge of the national monitoring, according to a periodicity set by AFSSAPS. They include a descriptive analysis of all the data collected during the ATU validity period (data collected since the previous report and cumulated data) in the context of the protocol for therapeutic use and information collection, as well as any new relevant information on the medicinal product since the cohort ATU was granted, particularly as regards actual conditions of use and safety.

Reimbursement of ATU-granted medicines

For ATUs that are followed by a MA, different reimbursement conditions apply according to 3 periods (period between ATU and notification of MA, period between notification of MA and entering into force of MA, period between entering into force of MA and publication of the MA)[20].

The following conditions solely concern the period between ATU and notification of MA (for all cohort ATUs and possible for nominative ATUs) or ATU that is not followed by a MA (most nominative ATUs).

In order to buy and supply ATU drugs, the drug needs to be registered on a list of products 'agréés à l'usage des collectivités". ATU drugs are solely available in hospital pharmacies or other diverse public health institutions but can be provided to hospitalised as well as outpatients (retrocession).

Drugs with a cohort or nominative ATU not classified for hospital use only and for outpatients are included in the reassigned list (articles R. 5126-103 et R. 5126-104 of the public health Code - rétrocession). These reassigned ATU drugs are reimbursed at 100% by the health care insurance on the basis of their transfer price (prix de cession). This price comprises the purchase price (based on price convention between the hospital and the pharmaceutical laboratory) + lump sum margin for the costs of administration and distribution by the hospital + VAT.[21]

19 for the composition of this Committee see: http://www.afssaps.fr/Activites/Autorisations-de-mise-sur-le-marche/Commission-nationale-d-autorisation-de-mise-sur-le-marche/(offset)/7

20 for more details see http://www.sante.gouv.fr/adm/dagpb/bo/2007/07-05/a0050112.htm

21 http://www.sante-sports.gouv.fr/IMG/pdf/medicaments-2.pdf

When drugs are administered to a hospitalised patient, the cost of any cohort or nominative ATU drugs is met by the health establishment treating the patient. This expenditure is covered by the establishment's financial allowance for missions of general interest and by assistance for contracting. This allowance can be increased to cover exceptional or unanticipated expenditure related to the purchase of ATU drugs.

7.1.2 Rare and chronic diseases in France

The French government elaborated an ambitious National Plan for Rare Diseases 2005-2008 (Plan National Maladies Rares 2005-2008)[33]. The Plan has the general aim to improve the equal access to healthcare services for rare disease patients (from the diagnosis phase to the reimbursement of costs) and puts forward following 10 priorities, amongst others:

- A deeper knowledge of the epidemiology of rare diseases;
- Recognition of the specificity of rare diseases;
- Dispersion of information to the general public and healthcare providers;
- Education of healthcare providers on the diagnosis of rare diseases;
- Improvement of the quality of care;
- Investments in research on orphan drugs;
- Etc.

One of the priorities is the establishment of about centres of reference who were awarded the label by the Minister of Health for five years. The centres have a double role: they intervene as expert centre for one or more rare diseases and they are the resource centre for patients coming from outside the region. The French government budget €100 millions (spread over 5 years) to realize the plan.

In France, the level of reimbursement of patients suffering from a rare disease is strongly linked to the recognition of their disease as a chronic disease.

Recognition of chronic diseases (Affectation de longue durée - ALD) is defined by the Social Security Code (Article L 324 du Code de la Sécurité Sociale). The patient's referent GP (médecin référent) diagnoses the chronic disease and elaborates a so-called 'treatment protocol', describing the pathology and prescribing the required treatment. Finally, the medical committee of the public health insurance fund (Caisse d'Assurance Maladie) takes a decision, based on the request for recognition that was submitted by the referent GP.

People that suffer from a chronic disease are exempted from cost-sharing and thus fully reimbursed by the public health insurance fund (called exoneration du ticket modérateur). However, this is only true for healthcare expenses that are linked to the treatment of their chronic disease and not for any other disease. The exemption period from cost-sharing is mentioned by the decision of the Medical Council of the Health Insurance Fund (Caisse d'Assurance Maladie) and can be extended.

In France, three categories of chronic diseases can be distinguished:

- Chronic diseases that are listed as 'ALD 30', or diseases that require an extend period of treatment and an expensive treatment. The list of these 30 chronic diseases are defined and adapted by the Minister of Health (e.g. article L. 322-3-3° et article D. 322.1 du Code de La Sécurité Sociale/décret n° 2004 - 1049 du 4 Octobre 2004[34]). The majority of 'orphan diseases' (maladies orphelines) are considered as 'ALD 30' and appear directly or indirectly on this list (see annex 9.14.4).
- Chronic diseases that are not listed as 'ALD 30', but that are recognised as a chronic disease (Affections 'Hors liste, article L. 322-3-4° du Code de la Sécurité Sociale). It concerns diseases that require a period of treatment that is exceeding 6 months as well as an expensive treatment. Examples of affections 'hors liste' are :
  - malformation congénitale des membres ;

- embolie pulmonaire à répétition ;
- dégénérescence musculaire ;
- asthme;
- etc.

- Polypathologies, when a patient suffers from more than one disease, resulting in a state of invalidity and requiring a period of treatment that is exceeding 6 months. For instance, a patient suffering from blindness, while at the same carrying the consequences of a hip fracture (article L. 322-3-4° du Code de la Sécurité Sociale).

For some specific rare diseases, a number of 'simple', pharmaceutical products that are used by rare disease patients are not (fully) reimbursed by the public health insurer, even if they are reimbursable if they are prescribed for other diseases. A good example is vitamins that are prescribed to mucoviscidosis patients.

Article 56 of Social Security Finance Law (Loi de Financement de Sécurité Sociale, 21 décembre 2006[35]) enables the exceptional and temporary reimbursement (renewable period of 3 years) of certain non-reimbursed prescribed drugs, products or treatments for chronic or rare diseases. The following conditions have to be fulfilled:

- There is no other, appropriate alternative available within the conventional reimbursement system;
- The use of the product or treatment is primordial for the health status of the patient or necessary to avoid aggravation
- The product or treatment has been subject to a recommendation or advice within (6 months) of the Haute Autorité de Santé (HAS) who asks systematically for the advice of AFSSAPS

Applications for a recommendation by the HAS can be introduced by the minister of Health, the minister of social security or the "conseil de l'Union nationale des caisses d'assurance-maladie.

Decree n° 2008-211[36] concretizes the conditions under which the HAS needs to treat such requests and the time frame that should be respected with respect to a final decision[22].

7.1.3 Recent Health French System Reforms

Cost control is a key issue in the French health system, as the health insurance scheme has faced large deficits for the last 20 years. More recently the deficit has fallen, from €10-12 billion per year in 2003 to a €6 billion in 2007. This may be attributed to the following changes, which have taken place in the last two years:

- A reduction in the number of acute hospital beds
- Limits on the number of drugs reimbursed; around 600 drugs have been removed from public reimbursement in the last few years
- An increase in generic prescribing and the use of over the counter drugs
- The introduction of a voluntary gate keeping system in primary care
- Protocols for the management of chronic conditions
- From 2008, new co-payments for prescription drugs, doctor visits and ambulance transport will not be reimbursable by complementary private health insurance

Recent health policy developments include:

- Act of 13 August 2004 on the health insurance reform, based on three main pillars:

22 http://www.has-sante.fr/portail/upload/docs/application/pdf/2009-06/guide_methodo_art56_web.pdf

  - The rationalization of healthcare: obligation to choose a regular GP, personalized medical files and introduction of a new Carte Vitale (a smart card containing the health beneficiary's details) from 2007 to 2010.
  - A complementary contribution by each beneficiary: contribution of one non-reimbursable euro on each visit to a GP or specialist and flat-rate hospital contribution of €15.
  - Combating fraud: more stringent checks on sick leave and Carte Vitale bearing an ID photo.
- Act of 9 August 2004 on the public health policy, which introduces five major five-year programmes and regional public health policy management. The five programmes for 2004-2008 are:
  - The cancer plan,
  - The plan to combat violence, abuse, risk behaviour and addictive behaviour,
  - The plan to curb the impact of environmental factors on health,
  - The plan to improve the quality of life of patients with chronic illnesses,
  - The plan to improve treatment and care for patients with rare diseases.

Keypoints France

- **In France, the distinction is made between universal, public health insurance, providing a standard benefits package for all residents (assurance maladie obligatoire) through large occupation-based funds, and complementary private health insurance.**
- **The principle of cost-sharing where patient are carrying a part of the cost burden, or ticket modérateur, is extensively applied to public-financed health services in France. However, there are safety nets for categories of citizens that are exempt from co-insurance regulations and that are 100% reimbursed: this applies for people with invalidity or with work injury, people with specific chronic illnesses and low income patients.**
- **Health insurers have a public and statutory character with a membership based on occupation. There is no competition and no system of risk adjustment provided, even though there is inadvertent risk selection based on occupation.**
- **Healthcare providers (extra muros and intra muros) charge their delivered services in function of two elements: the classification (nomenclature) of groups of services based on their nature, and the negotiated price for these different service groups. The Nomenclature lists also all medicines that are reimbursed by public health insurance to the patient.**
- **The French government elaborated an ambitious National Plan for Rare Diseases 2005-2008. The Plan has the general aim to improve the equal access to healthcare services for rare disease patients (from the diagnosis phase to the reimbursement of costs).**
- **In France, the level of reimbursement of patients suffering from a rare disease is strongly linked to the recognition of their disease as a chronic disease. People that suffer from a chronic disease are exempted from cost-sharing and thus fully reimbursed by the public health insurance fund. This is only true for healthcare costs linked to the treatment of their chronic disease and not for any other disease they might have.**

- **The French healthcare system recognizes three categories of chronic diseases: 1) Chronic diseases that are listed as 'ALD 30', or diseases that require an extend period of treatment and an expensive treatment. The list of these 30 chronic diseases are defined and adapted by the Minister of Health; 2) Chronic diseases that are not listed as 'ALD 30', but that are recognised as a chronic disease as they require a period of treatment that is exceeding 6 months as well as an expensive treatment; 3) Polypathologies, or in case a patient suffers from more than one disease, resulting in a state of invalidity and requiring a period of treatment that is exceeding 6 months.**
- **Temporary authorisation (ATU) for the use of new drugs outside of the framework of clinical trials, without Marketing authorisation (MA) in France, whether they benefit or not from a MA abroad, at individual patient level (at the request of the prescribing physician) or homogeneous group (cohort) level (at the request of the pharmaceutical firm) can be granted by AFSSAPS in a very short time laps. Solely drugs that are used for the treatment of serious or rare diseases, for which there is no other treatment available and cost-benefit analysis of the treatment was conducted, with a positive result are eligible for ATU.**
- **Article 56 of Social Security Finance Law enables the exceptional and temporary reimbursements of certain drugs, products or treatments for rare or chronic diseases, if there is no other, appropriate alternative available within the conventional reimbursement system and if the product or treatment has been subject to a recommendation or advice of the Haute Autorité de Santé (HAS)**

7.2 THE NETHERLANDS

An introduction of the Dutch healthcare system and the organisation of the healthcare provision can be found in annex. 9.15.

7.2.1 Reimbursement of medicines

7.2.1.1 Extra muros vs. intra muros

In order to get an idea about the reimbursement of medical care, one should make the distinction between healthcare provision **intra muros** (within the hospital) or **extra muros** (outside the hospital). Within the context of medicines, intra muros is not necessarily being considered as period of hospitalisation. Many medicines, in particular orphan drugs or expensive drugs, are prescribed and provided by hospital specialists without the patient being hospitalised.

Table 11: Overview of extramural and intramural reimbursement

	Extramural	Intramural
System of reimbursement	Reimbursed to the patient under the **Drug reimbursement system** *(GVS–Geneesmid-delenvergoedingssysteem)* GVS has 3 annexes: - *Schedule 1A:* medicines for which an alternative exist that is 100 % reimbursable. Maximum limit of reimbursement/patients has to pay an absolute amount. - *Schedule 1B:* medicines that are 100% reimbursable. - *Schedule 2:* medicines which require an authorisation for (partial of full) reimbursement	Costs are charged on the hospital general budget, not to the patient. Compensation arrangements: hospitals are extra compensated by health insurers for specific medicines and under specific conditions under: - Policy Regulation on 'Expensive Drugs' - Policy Regulation on 'Orphan Drugs' (only academic hospitals)
Who decides on reimbursement of medicines? / Parties involved?	**College of Health Insurers** (CVZ-*College van Zorgverzekeraars*) advises the Government on therapeutic value and cost of medicines Dutch Government- **Minister of Health** finally decides on the adoption of medicines on the list of Drug Reimbursement System	Step 1: Framework set by the supervising, independent Dutch Healthcare Authority (*Nederlandse Zorgautoriteit*): - Members of Health Insurers - Members of Healthcare Providers Step 2: Bilateral negotiations between: - Health Insurers - Healthcare providers Exception: College of Health Insurers also advises the Dutch Healthcare Authority on the adoption of medicines within the policy regulations 'Orphan Drugs' and 'Expensive Drugs'

Intra muros

The costs for (orphan) Drugs that are given to patients **intra muros**, are charged on the hospital budget. Hospitals are compensated by the health insurers for the medicines they are prescribing, based on negotiations between both parties. The framework, in which negotiations on compensations and budget between hospitals and health insurers take place, is designed and supervised by the Dutch Care Authority (Nederlandse Zorgautoriteit), a non-governmental, independent board that takes up the role of supervisor and regulator of the healthcare market in the Netherlands.

Because the expensive (orphan) drugs, prescribed intra muros, laid a heavy burden on the budget of hospitals in the past, compensation arrangements (beleidsregels) have been concluded between the health insurers and healthcare providers, applying for specific medicines and under specific conditions. The (partial) compensation of hospitals by the health insurer is based on an ex-post calculation. Two compensation arrangements are relevant from the perspective of rare disease:

- Policy Regulation on Expensive Drugs CI-1087, 1st of January 2009: under this policy regulation, hospitals will be compensated for a registered drug by the health insurer, if the estimated total costs correspond to 0.5% or more of the total medicines expenditure of hospitals at macro-level. If this condition is being met, a hospital will be reimbursed 80% of the costs, based on a ex-post calculation.
- Policy Regulation on Orphan Drugs CI-1043: only applying to academic hospitals: academic hospitals are eligible for compensation, if the total costs of an orphan drug are estimated to exceed 5% or more of average total medicines expenditure of academic hospitals. If this condition is being met, the health insurer will fully (100%) reimburse the costs of the orphan drugs on top of the regular budget

Hospitals (and manufacturers) can apply for additional funding at the Dutch Care Authority (Nederlandse Zorgautoriteit). Following, the Dutch Healthcare Insurance Board (HCIB-College voor Zorgverzekeraars, CVZ) takes a decision based on the submitted dossier by the applicant. Additional funding is in any case conditional. After three years, the adoption of the medicine and its funding will be reassessed, based on new evidence and research on outcomes.

For intramural drugs, the relevant criteria for being temporarily listing as an orphan drugs or an expensive are:

- the therapeutic value
- cost prognosis
- cost-effectiveness indication
- the proposal for outcomes research

After three years, the listing of intramural drugs is reassessed, based on the following criteria:

- the therapeutic value
- the actual costs of the medical product
- the cost-effectiveness
- the efficient prescription, based on outcomes research.

Extra muros

Outside the hospital (**extra muros** treatment), patients rely on the services of the pharmacist, doctor or specialist to receive treatment of medicines. For these treatments or products, other reimbursement rules are applicable.

If a medicine is registered and is granted Marketing Authorisation, the Dutch Healthcare Insurance Board (HCIB, College van Zorgverzekeraars-CVZ) will advise the Dutch government on the adoption of the product on the list of the Drug Reimbursement System (GVS, geneesmiddelen-vergoedingssysteem).

Medicines that are on the list of GVS are compulsory reimbursed by the health insurance providers and fall under the standard benefits package. However, the Drug Reimbursement System distinguishes between different categories of medicines, from a reimbursement point of view:

- Schedule 1A: medicines for which an alternative exist that is 100 % reimbursable. Maximum limit of reimbursement/patients has to pay an absolute amount.
- Schedule 1B: medicines that are 100% reimbursable.
- Schedule 2: medicines which require an authorization, in order to be (partial of full) reimbursable

The assessment criteria of medicines for extramural and intramural drugs are relatively similar. Regarding to extra muros treatment, the HCIB judges on:

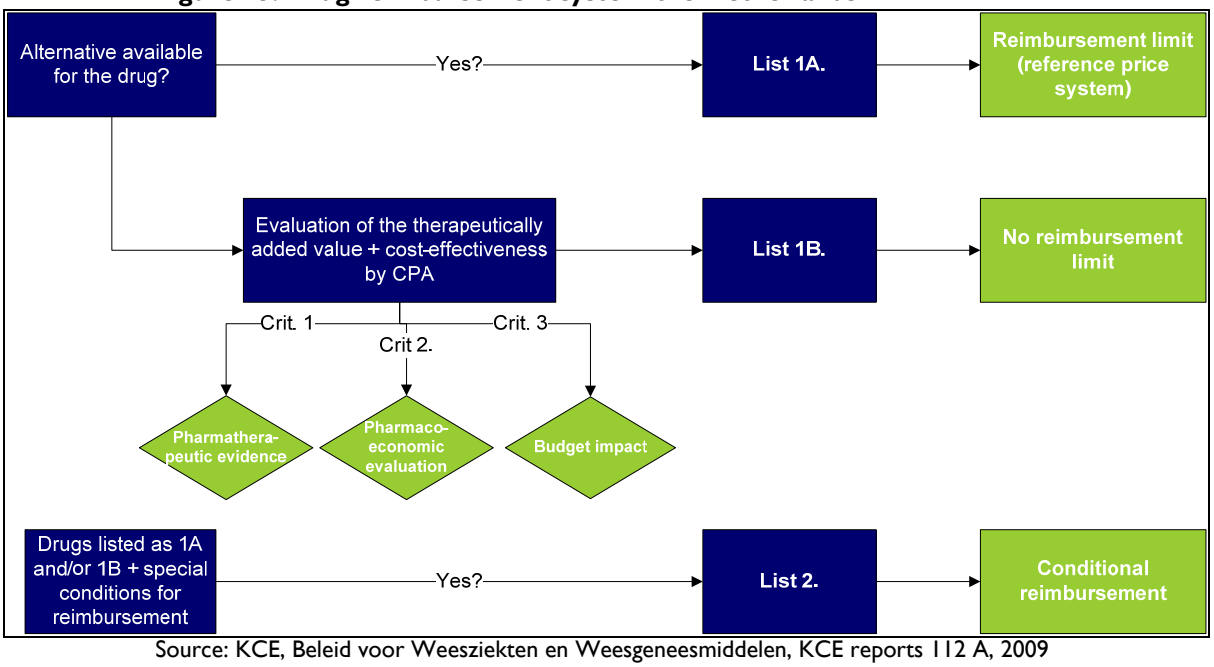
- The therapeutic value of the drug, based on a comparison with the existing standard treatment
- Cost-effectiveness and budgetary impact.

The assessment and decisional procedure followed by the final decision of the Minister of Health can take 90 days, in accordance to the EU transparency regulation. The assessment is done by the Committee for Pharmaceutical Aid (CPA) of the HCIB.

In case of non-interchangeable drugs, the procedure is the same for orphan and non-orphan drugs and is based on three components:

- the pharmacotherapeutic (therapeutic value) evidence
- the pharmaco-economic evaluation (cost-effectiveness)
- the budget impact.

Figure 13: Drug reimbursement system the Netherlands



Source: KCE, Beleid voor Weesziekten en Weesgeneesmiddelen, KCE reports 112 A, 2009

For EU-registered orphan drugs for which a Market Authorisation exists, but that are not listed in the GVS - for example because they are still in an experimental stage or due to a lack of 'added therapeutic value' –, the Minister of health developed a specific subsidy programme for the reimbursement of orphan drugs that are prescribed extra muros. The aim of the subsidy programme is to ensure that the assessment procedure to which an orphan drug is imposed, does not impede the reimbursement of the orphan drug.

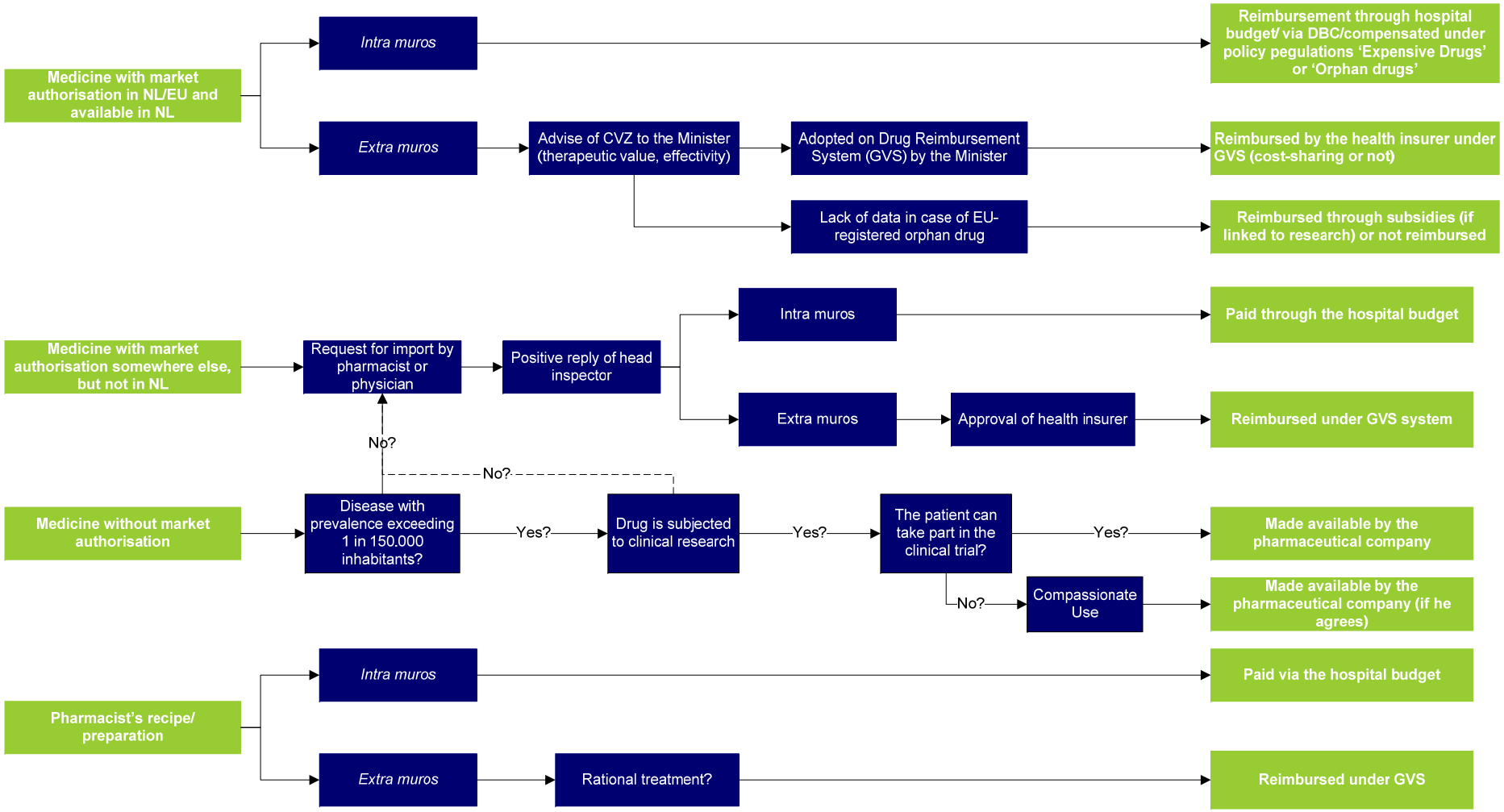
Medicines that have been granted Market Authorization somewhere else, but not in the Netherlands, require a positive reply of the Head Inspector on an import request by a healthcare provider. In case of a positive advice, reimbursement takes place following the intra muros or extra muros provisions.

For drugs with no Market Authorisation, one could distinguish different options:

- An insured suffers from a disease with prevalence in the Netherlands not exceeding 1 in 150.000 inhabitants and the prescription of a non-registered drug is considered as 'rational': the same reimbursements rules are applicable, as for drugs with non-Dutch Market Authorization.
- An insured suffers from a disease with prevalence in the Netherlands exceeding 1 in 150.000 inhabitants:
  - The drug is not subjected to clinical research: the same reimbursements rules could be applicable, as for drugs with a non-Dutch Market Authorization.
  - The drug is subjected to clinical research, in which the insured could take part. After the clinical trial, pharmaceutical companies often continue to provide these drugs to the test persons if the drug turned out to be effective.
  - Finally, if the insured does not meet the criteria for participation in clinical research, he could rely on a 'compassionate use' programme in exceptional cases. Compassionate use is possible if:
  - There is a declaration of the healthcare provider (GP, specialist...);
- There is no alternative drug on the market and is waiting a MA.

The following figure illustrates the different reimbursement options for a medicine for rare diseases.

Figure 14: Reimbursement of medicines for rare diseases in the Netherlands



Source: Steering Committee Orphan Drugs, Beschikbaarheid van Geneesmiddelen voor Zeldzame Ziekten, 2009

Since the reform of the healthcare and insurance system, there have been many cases of disputes between patients, hospitals, insurance providers, pharmaceutical companies and the government with respect to the reimbursement of expensive pharmaceutics or treatment for life-threatening diseases. The Dutch Authority of Health Insurers, the entity that advises the Ministry with respect to the reimbursement of pharmaceuticals, registered 1.206 disputes in 2004, in comparison to 772 disputes in 2003. Pharmaceutical companies started seven legal proceedings against the Dutch state in order to have some 'expensive' pharmaceutics adopted by the reimbursement system for pharmaceutics.

Regarding rare diseases, it is relevant to mention the Steering Committee Orphan Drugs (Stuurgroep voor Weesgeneesmiddelen), which was established in 2001 in order "to encourage the development of orphan drugs and to improve the situation of patients with a rare disease, especially to strengthen the transfer of information on rare diseases".

Keypoints Netherlands

- **The Dutch health insurance system distinguishes three components: statutory health insurance for exceptional medical expenses, covering a wide range of chronic and mental healthcare services with an exceptionally high cost; statutory basic health insurance, providing a standard benefits package; and complementary health insurance, covering less vital healthcare services that are not covered by the standard benefits package.**
- **With the 2004 reform of the healthcare system, the Dutch government introduced a so-called 'regulated competition' among health insurers, based on the idea that competition among health insurers (but also among healthcare providers) would decrease healthcare expenditure and result in a more cost-efficient healthcare provision. As the healthcare providers and insurers are operating in a system where they have to negotiate with each other on price and content of medical care, the role of the Government has evolved towards supervision and setting frameworks.**
- **Health insurers in the Netherlands are private actors and are allowed to make profits. With respect to the standard benefits package, they are bound by acceptance obligations, which means they cannot refuse patients or patient groups as becoming their clients. In exchange for the acceptance obligations, the Dutch government compensates the risks that health insurers face of having a higher number of risk patient groups in their clients through a risk compensation system, or "risicovereveningssysteem" in Dutch. The definition and categorization of risk patient groups is not explicitly limited to rare diseases, but is broadly defined to all costly chronic diseases.**
- **The distinction between healthcare provision intra muros (within the hospital) or extra muros (outside the hospital) is utmost relevant regarding the reimbursement of medicines.**
- **Costs for (orphan) drugs that are given to patients intra muros, are charged on the hospital budget. The framework, in which negotiations on compensations and budget between hospitals and health insurers take place, is designed and supervised by the Dutch Care Authority (Nederlandse Zorgautoriteit). Specific compensations arrangements (beleidsregels) have been concluded between the health insurers and healthcare providers, applying for specific medicines and under specific conditions. Two compensation arrangements are relevant from the perspective of rare disease: the Policy Regulation on Expensive Drugs CI-1087 (for all hospitals) and Policy Regulation on Orphan Drugs (only for academic hospitals).**

- **Regarding treatment outside the hospital (extra muros), medicines that are listed under the Drug Reimbursement System are compulsory reimbursed by the health insurance providers under the standard benefits package. The Drug Reimbursement System distinguishes between different categories of medicines, regarding their reimbursement: Schedule 1A: medicines for which an alternative exist that is 100 % reimbursable. Maximum limit of reimbursement/patients has to pay an absolute amount; Schedule 1B: medicines that are 100% reimbursable; Schedule 2: medicines which require an authorization, in order to be (partially of fully) reimbursable**

7.3 SPAIN

An introduction to the Spanish healthcare system and the organization of healthcare provision can be found in annex 9.16.

7.3.1 Rare diseases in Spain – coverage of costs of treatment and medication

In Spain there is at this moment no specific framework for the treatment of patients with rare diseases. The medical and pharmaceutical treatment is part of the global NHS system and medical care as well as drug prescription and the use of medical devices is regulated by in the overall 'cartera de servicios'. If a treatment or medication needed for the treatment of patients with rare diseases is not included in this 'cartera', normally the costs are not financed by the NHS.

The last decade different initiatives have been taken to improve the care to patients with rare diseases. Till now a global plan for rare diseases does not yet exist at national level. At regional level, several Autonomous Communities developed their own regional plans but these are not integrated. Even if some of these plans provide additional coverage of treatments and medication for patients with rare diseases, these are only accessible for the citizens living in these specific regions.[37-39] As such these regional plans lead to unequal care provision. In some regions certain types of medication, devices or products will be included in the regional 'cartera de servicios' and be provided free of charges at hospital level or with (limited) co-payment if delivered at local pharmacies whereas in other regions patients will have to pay for them. Such plans or approaches at this moment have been introduced in the regions of Andalucia, Extremadura, Cataluña and País Vasco (Plan de Genética). In other regions rare diseases are not even mentioned as an issue in the regional health plans.

In 2007, the Spanish Senate adopted a resolution to set up a study to analyze the specific situation of patients with rare diseases and to examine gaps in the NHS. Very recently, during the last month of June, the Spanish Ministry of Health launched a 'strategy on rare diseases'.[40] The aim is to improve knowledge on rare diseases, improve accessibility of care and coverage of costs of treatments and medication in the NHS.

7.3.1.1 Reference centers

One of the measures that were taken to improve the provision of medical care and assistance for the treatment of rare diseases or special (exceptional) care is the creation of 'Reference centers' (CSUR – Centros, Servicios o Unidades de referencia). Till now more than 80 such reference centers have been recognized and integrated into the NHS. In principle access to these centers has to be guaranteed to all Spanish citizens. These reference centers sometime can be very distant from the home location of the patient. To overcome this obstacle, special provisions for travel costs have been introduced. Since the reference centers are part of the NHS, treatment as well as medication is to be provided at no cost for the patients.[41]

7.3.2 Access to medication in special situations

Next to the creation of the reference centers, at national level new legislation has been passed to improve access to medication in 'special situations'. [42]

This ruling provides access to investigational drugs to patients who have no successful treatment and suffer from a serious disease or have a life-threatening situation. The authorization is given by the Spanish Agency of Medicines (AEMPS).

7.3.2.1 Use of medication in pre-market authorization stage

The Spanish Agency of Medicines and Health Products (AEMPS) may authorize the prescription and the use of drugs that have not yet been accepted and authorized in Spain, for individual patients that suffer from a chronic or a serious disease or are in a life threatening situation without any satisfactory therapeutic alternative available. It concerns patients which are not part of a clinical trial and who are in a clinical situation that cannot wait till the investigation on the new treatment will be completed. These drugs have to be subject of a procedure for acceptance or be part of a clinical trial procedure (but meant for patients that are no part of the clinical trial). Access to these drugs may be authorized individually for a patient (compassionate use), or relying on a temporary authorization for use for a group of patients. If an individual authorization is asked, the hospital where the patient is treated will have to enter the request. The AEMPS has to decide on the petition within a period of 10 days. If the decision is negative the hospital has a period of 10 days to add information and argue the case.

Next to the individual authorization, a temporary authorization procedure has been foreseen for drugs that are not accepted and authorized yet and that are meant for a (homogeneous) group of patients. The pharmaceutical company (or the promoter of a clinical trial) can ask to obtain such authorization if the drug is subject to an acceptance procedure that has not been accomplished yet or if the drug is subject to a clinical trial procedure. The authorization can be given for the use of this drug to a specified group of patients (that, if a clinical trial is running, are not included in this trial). The hospital where the drug will be administered has to guarantee the patient meets the conditions that have been specified by the AEMPS. Prescribing and using these drugs is restricted to the specialized care level (hospital care but also ambulatory treatments in hospitals).

More detailed information on the evaluation criteria by the AEMPS could not be obtained.

7.3.2.2 Off label use

The new regulation also includes the possibility for the use of drugs for different indications as those for which they have been authorized (off label use). The authorization for off label use is not an individual authorization (not for one specific patient) but for homogeneous groups of patients. The prescribing doctor will have to justify the off label use in the individual medical file of the patient but does not have to ask for an individual authorization from the AEMPS. The AEMPS may issue recommendations to be taken into account in the therapeutic-care protocols developed by health centers. This may be especially relevant in therapeutic areas in which research activity is intense and the pace of evolution of scientific knowledge may precede the steps necessary to incorporate such changes in the technical file of the drug. It allows the use of drugs for conditions that have not been established in the authorization of the medicine, often by absence of commercial interest for the pharmaceutical companies to accomplish the studies necessary to obtain the authorization of the AEMPS.

7.3.2.3 Foreign drugs

The new regulation facilitates the access to drugs that are not authorized (yet) in Spain and that are commercialized in other foreign countries in cases where the use of these drugs is essential for the medical treatment of a patient with a serious disease. This creates the possibility of authorizing the import as well as prescribing and administrating them in Spain, whenever the drugs are legally authorized in other countries. Such authorization is given when using these drugs is essential for the prevention, the diagnosis or the treatment of concrete pathologies and where there does not exist a suitable alternative for the treatment of the patient that has been authorized for use in Spain. Again the procedure foresees as well an individual authorization for one patient as well as a non individualized authorization for a specific (homogeneous) group of patients. The AEMPS can issue protocols in which the use of the drug is conditioned.

7.3.3 Overall situation for drugs and medical devices used in the specialized care

As explained previously for drugs that are used at specialized care level (used in NHS hospital care and prescribed by a medical specialist) there is no cost for the patient. Costs of drugs in hospitals are financed in the hospital budget. The hospital budgets resort under the competence of the Autonomous Communities and determined by the Regional Health Services.

If the use of the drug is authorized by the AEMPS, the drug can be used at hospital level. This counts for all medication authorized by the AEMPS, as well as medication that is part of the 'cartera de servicios' as for drugs authorized in special situations and orphan drugs. One could conclude that this would mean all authorized medication is covered by the NHS.

One big restriction on the availability and the use of drugs that are not integrated in the 'cartera de servicios' of the NHS or the Regional Health Service is that the hospital will decide on provision of the drug within the hospital. Since these drugs have to be covered by the hospitals budget, the hospitals decide on the use of them. In practice hospitals have a Pharmacy and Therapeutics Committee (on drugs) (Comisión de farmacia y terapéutica) as well as on medical devices (Comisión de evaluación de técnicas sanitarias). These committees decide on the use and the conditions for using the drugs and the medical techniques and devices. In some autonomous communities such committees are organized at regional level, preventing differences within the different hospitals of the region. Such for example is the case for the region of Andalusia.

This situation leads to differences in drug provision between the regions (and in some regions between hospitals of the same region) and does not match with equity. The fact the management of the hospital has a great impact, on availability of drugs for serious diseases, is the reason why the Spanish Organisation of Rare Diseases asked to change the actual regulation eliminating the decision at hospital level and enhancing the position of the prescribing medical specialist.[43]

Keypoints Spain

- **Organization of the Spanish health care system is rather complicated. Competences are divided between the national level and the autonomous communities.**
- **A minimum health care provision is guaranteed (as regulated at national level within the National healthcare System). This minimum comprehends primary care, specialised care and pharmaceutical products.**
- **At Regional level this minimum provision of health care services can be expanded. This additional health care provision is however not financed by the NHS. The regions have full competences but have to support financing them on their own.**
- **The ambulatory care within the NHS is organized through the primary care centers were patients see their general practitioner, nursing staff and were, depending of the size of the centre, basic technical medical exams can be performed.**
- **Hospital care is organized through a network of public and private hospitals. The NHS has agreements with certain private hospitals. These agreements make these private hospitals accessible for patients under the NHS system.**
- **Medication as well as medical techniques or devices that are financed through the NHS are included in the 'Cartera de servicios comunes de prestación farmacéutica' and for hospital care in the 'cartera de servicios communes de atención especializada'. Authorisation and acceptance is regulated by the AEMPS (Agencia Española de Medicamentos y Productos Sanitarios). Acceptance and financing under the NHS is decided by the Ministry of Health in accordance with the 'Consejo Interterritorial del Systema Nacional de Salud'.**
- **Medication and medical devices that are not included in the Service portfolio of the NHS (Cartera de servicios), are not financed under the NHS. The autonomous regions can expand this service portfolio but, if they do so, they have to finance the costs themselves.**
- **For medication that is distributed at primary care level through the local pharmacies, the patient has to pay a part of the costs (co-payment). It varies from 40% to 0% depending from the type of drugs and the personal situation of the patient.**
- **Medication and medical devices that are used at hospital level are included in the hospital budget. Costs of medication are not charged to the patient.**
- **If the medication or the devices are included in the 'cartera de servicios' from the NHS, these are available if prescribed by a medical specialist. For medication or medical devices not included in the 'Cartera de servicios' of the NHS, decisions on the use of them are taken at hospital level. The policies on this varies between the Autonomous Communities since they can expand the 'cartera de servicios' for their region.**
- **Spain has no specific national plan for rare diseases at national level. Several Autonomous Communities did develop their own plan or framework.**
- **Specialised reference centres for rare diseases and exceptional care are being established in cooperation between the national and the regional authorities. These are to be equally accessible for al Spanish citizens and are covered by the NHS.**
- **Spain recently passed new legislation on the access to medication in special situations, the use of medication in pre-market authorisation stage, the use in conditions other than those authorised and for foreign drugs. Acceptance is organised at central level by the AEMPS.**

7.4 OVERVIEW OF THE CASES

As described in the chapter on the methodology, the research team and the leading medical officer of the SSF developed a list of cases as an alternative approach for the international exploration. For these cases, we looked on how the costs of the corresponding drugs or devices are financed in The Netherlands, France and Spain.

The details of the outcome are included as annex 9.17 to the report. The table below (table 12) gives an overview of the reimbursement of these products. Some parallels can be drawn. The products that are reimbursed or mostly reimbursement (or financing in the budget of the hospital) are drugs and products prescribed and administrated within the hospital.

For the drugs Vidaza®, Kuvan®, Mabthera®, Avastin®, Flolan®, Remodulin® (not found in Spain), Revatio®, Imoduline® and Cystadane® financing is comparable in all these countries. Slight differences occur on eventual co-payment when ambulatory use is also being reimbursed.

Myozine® is financed for hospital use in the Netherlands and Spain and was not found in the nomenclature in France. In the Netherlands there is reimbursement of 60% when the drug is prescribed by a medical specialist for ambulatory use. Ditropan® was not found in the Netherlands and is financed for hospital use in Spain and France. Contrathion® was only found in France and is fully financed for hospital use.

In Spain implants in general are included in the hospital budget; there is no individual billing to the patients. However this does not guarantee that the implants are available and used since the hospitals (and indirectly the medical profession) decides whether and in what situation they will be used.

Table 12: overview of cases in the Netherlands, Spain and France

		Netherlands	Spain	France
Name	Activ sustance	Reimbursement	Reimbursement	Reimbursement
Adagen	Pegademase bovine	US-registered orphan drugs (non-EU): not reimbursed intra muros, nor extra muros. Today, adagen could possibly be paid by pharmacist's, pharma company or the hospital through ad hoc-reimbursement systems (however not by the health insurers) **GREY ZONE.**		Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)
Vidaza	Azacitidine	Received recently a positive advice to be recognized as 'Expensive Drugs' (intra muros) - 100% reimbursable - prescribed by specialists in all hospitals	Hospital use Financed NHS without any co-payment of the patient	When prescribed in the hospital 100% financed also for ambulatory patients
Kuvan	sapropterin dihydrochloride	Received recently a positive advice to be adopted in the Drug Reimbursement System (GVS-extra muros) - 100% reimbursable	Hospital use Financed NHS without any co-payment of the patient	When prescribed in the hospital 100% financed also for ambulatory patients 60%
Cellcept	Mycophenolate mofetil	Reimbursed under GVS - Schedule 2 (advies CVZ, 2007)	Diagnose by medical specialist Co-payment of the patient 10% max.2,64€	CIP: depending on the product; Homologation Assurés Sociaux (A) - 100% - since 10/01/2006 (re-examined in 2013) + Homologation Collectivité C: since 10/01/2006
Mabthera	Rituximab	Recognized as 'Expensive Drugs' (intra muros) - 100% reimbursable - prescribed by specialists in all hospitals	Hospital use Financed NHS without any co-payment of the patient	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités C: since 1998
Avastin	Bevacizumab	Recognized as 'Expensive Drugs' (intra muros) - 100% reimbursable - prescribed by specialists in all hospitals	Hospital use Financed NHS without any co-payment of the patient	When used in the hospital 100% financed not for ambulatory patients
Bi-myconase and/or sucraid	glucamylase & inver-tase (saccharase)			Not found in the nomenclature
Ointmenst, disinfectants, pain-killers, tape, food supplements				Not found in the nomenclature
Implantable cardiac valves (aortic valves)			In hospital's budget	Not found in the nomenclature
Implantable pulmonary valves			In hospital's budget	Not found in the nomenclature
Brainstem implant		Probably not reimbursed under GVS - Advies CVZ	In hospital's budget	Not found in the nomenclature
Neuro stimulator of the stomach				Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)
Flolan	époprosténol	Reimbursed under GVS - listed under Schedule 2 + Paid on the hospital budget (intra muros)	Hospital use Financed NHS without any co-payment of the patient	When used in the hospital 100% financed also for ambulatory patients
Remodulin	tréprostinil	Reimbursed under GVS - listed under Schedule 2 + Paid on the hospital budget (intra muros)		When used in the hospital 100% financed also for ambulatory patients
Viagra/revatio		Reimbursed under GVS - listed under Schedule 2 + Paid on the hospital budget (intra muros)	Hospital use Financed NHS without any co-payment of the patient	When used in the hospital 100% financed also for ambulatory patients
Ilomedine	iloprost	Reimbursed under GVS - listed under Schedule 2 + Paid on the hospital budget (intra muros)	Hospital use Financed NHS without any co-payment of the patient	When used in the hospital 100% financed
Elvorine				Not found in the nomenclature
Calcort	déflazacort		Other product - on prescription for long time treatment co-payment of 40%	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)
Myozine	alglucosidase alfa	Paid on the hospital budget (intra muros): adopted on the list of 'Orphan Drugs' - 80 % reimbursed - only prescribed by specialists in	Hospital use Financed NHS without any co-payment of the patient	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)
Sovrima	idebenone	Probably not reimbursed under GVS - Still in clinical trial process		Not found in the nomenclature
Ditropan for intravesical application	oxybutynin chloride		In hospital's budget	CIP: depending on the product; Homologation Assurés Sociaux (A) - 100% - sinds 10/01/2006 (reexamen in 2013) / Agrées aux collectivités: sinds 06/2006
implant cochléaire contralétéral (in fact bilatéral)			In hospital's budget	Not found in the nomenclature
Cranial implant on messure			In hospital's budget	Not found in the nomenclature
corticale electrodes			In hospital's budget	Not found in the nomenclature
intégra				Not found in the nomenclature
implant in mid ear			In hospital's budget	Not found in the nomenclature
diafragmatic pacemaker			In hospital's budget	Not found in the nomenclature
contrathion				When used in the hospital 100% financed
cystadane		Reimbursed under GVS - listed under Schedule 1B - 100% reimbursable	Hospital use Financed NHS without any co-payment of the patient	When prescribed in the hospital 100% financed also for ambulatory patients 60%

7.5 CONCLUSIONS OF THE CHAPTER ON THE INTERNATIONAL APPROACH

No mirror systems or comparable safety networks to the SSF exist in the countries studied.

7.5.1 Costs of drugs at secondary care level (hospital care)

As in Belgium the health care system differentiates primary care and secondary care (hospital care). Looking at the health care systems in the other countries that were part of the international approach in this study, one notices a major difference with the Belgian health care system on how costs of drugs and medical treatments are covered at hospital level. In Belgium there a third-party payer system for inpatient care and drugs, for which the sickness fund directly pays the provider while the patient only pays co-payments. If there is no coverage of the costs by the health insurance system, the costs are fully charged to the patient. There are some exceptions on these principles were hospitals are not allowed to charge specific costs to the patients. For example some antibiotics are paid at a flat rate to the hospitals and are not charged to the individual patient.

In all countries studied, medicines are included in the overall hospital budget. They are globally financed to the hospitals. In the Netherlands supplementary compensation (extra budget) for the hospital can be granted for expensive or orphan drugs. In Spain the hospital budget will be influenced by regional priorities (Autonomous regions) with possible differences between the regions.

Such a system of (closed or semi closed) hospital budgets including costs of drugs and medical devices leads to a different approach. It's the hospitals' responsibility to decide on the use of (authorized) drugs and medical devices. The costs are not charged to the patients. This could however lead to different approaches between hospitals and as such to different outcomes for patients. The specialist doctors have a joint responsibility at hospital level. Hospital care is not restricted to hospitalized patients but includes care to extra muros patients that are treated by medical specialist doctors in the hospital facilities.

7.5.2 Costs of drugs at primary care level

For primary care, costs of drugs delivered through the local pharmacies in the countries studied, are subject to (differentiated) co-payment. This is the same as in Belgium. Only drugs that are listed on the nomenclature code are reimbursed. In France there is no co-payment for patients with chronic disease if their disease has been recognized (ALD). In Spain the costs of drugs provided at the primary health care centers are not charged to the patients, only drugs delivered by the local pharmacies are subject to co-payment.

7.5.3 Reference centers for rare diseases

In both France as Spain health authorities foresee reference centers for treatment of rare and chronic diseases. Costs of treatments and drugs are included in the reference centers' budgets. In the Netherlands some hospitals are specialized in treating rare diseases. The health insurance companies contract these hospitals and as such influence concentration of patients in specialized hospitals. In Belgium till now every medical specialist can treat rare diseases and prescribe drugs or innovative treatments. The results from the SSF sample however reveal that a small number of hospitals present a large number of applications, which implies that "de facto" there is a kind of reference centre system for rare diseases in Belgium. There is however no regulation with regard to the systematic financing of the treatment in those particular hospitals.

7.5.4 Early access to new drugs

In all countries studied as well as in Belgium a system authorizing new drugs limits the provision and the reimbursement of new drugs. In Belgium registration and authorization of drugs is handled by the Federal Agency for drugs and Health products (FAGG – AFMPS). In Spain as well as in France and the Netherlands similar agencies exist. These agencies have a role on regulating access to drugs and devices.

In France as well as in Spain early access to new or innovative drugs can be asked for by medical specialists (for individual use) or by pharmaceutical companies (for use by group of patients). In France Authorizations for temporary use are granted by the AFSSAPS. Drugs used at hospital level are integrated in the hospital budget. These budgets are complemented if the hospitals treat patients that need expensive (or orphan) drugs. Drugs not classified for hospital use only and for outpatients are reimbursed at 100% by health care insurance on the basis on of their transfer price (prix de cession). This price comprises the purchase price (based on price convention between the hospital and the pharmaceutical laboratory) + lump sum margin for the costs of administration and distribution by the hospital + VAT. In Spain the AEMPS decides on the pre-acceptance stage, off label use and the use of foreign drugs (at individual patient level and for homogeneous groups). In Belgium early access to drugs is possible via the SSF (patient initiative). Early access is also possible within the framework of compassionate use (for drugs without MA in Belgium) or medical need programs (for drugs with an MA in Belgium for a given indication, but used for another indication). In contrast with the early access program of France that is financed by public means, these programs are set-up and paid by the pharmaceutical company, and the medicines are provided for free.

8 DISCUSSION AND CONCLUSIONS

The SSF was introduced in 1990 as an experiment, aiming at the creation of a safety net preventing people giving up high-cost, yet medically necessary care not reimbursed by the compulsory health insurance. A limitative set of objective -though susceptible to interpretation - eligibility criteria was defined by law. The action field of the SSF has gradually been broadened. Today, the question arises if the SSF meets the objectives that were initially aimed at and more particularly, if there are any unmet needs within the existing SSF framework as defined by law. This question, being part of the original approach of the project, has been abandoned because of methodological reasons. Indeed, it was impossible to identify and quantify people that theoretically meet the SSF reimbursement criteria but did not take up this opportunity. The aim of this study was to make an overall evaluation of the conceptual and procedural functioning of the SSF and to formulate suggestions for the optimization of these processes. In this section we give an overview of the answers to the research questions.

When drawing up conclusions, it is important to take the methodological limitations into account. The information on the organization of the SSF, its procedures and activities was obtained by a quantitative as well as a qualitative approach. Interviews were performed with a selection of employees of the NIHDI, with members of the College of medical directors and with the leading medical officer of the SSF. The most important limitations of this approach are that all interviewed persons are directly involved in the functioning of the SSF. On the other hand, a sample of the administrative data from the SSF database was analyzed. It does not constitute a random sample because solely the data of the patients who consented are included. Furthermore the database maintained at the SSF, containing the registration of the details of each application and decision, seems to be mainly targeted for operational use: to organize and register the day to day work flow and for the annual report. From this point of view, quite a lot of information on the applications and decisions is registered. However, from a scientific analysis point of view, the available information is registered in often insufficient detail or in a non-standardized way, precluding more in depth statistical analyses.

The evaluation of the functioning of the SSF focuses on the (interpretation of) the eligibility criteria for the reimbursement of costs, the clarity of these criteria for all parties involved, the information channels through which the stakeholders got to know the SSF, the appreciation of the applicable procedures and the decision-making process. Two methodological approaches were used to address these questions. On one hand qualitative surveys were performed to obtain a global overview of the perception of a sample of prescribing doctors, the social services and patients' associations. An important limitation is that solely stakeholders with a minimum experience with the SSF were interviewed and that only patients having introduced a file to the SSF were included. It is methodologically not feasible to isolate patients (or prescribing physicians) whose medical costs are potentially eligible for reimbursement by the SSF but did not introduce an application. Indeed, since the application field of the SSF is mainly determined by rare diseases or indication, there is little chance to find those patients (or prescribing physicians) in a random sample. On the prescriber side, solely physicians who consented to be interviewed were contacted. Interviews regarding specific topics were conducted with the umbrella organization Pharma.be. Moreover representatives of the pharmaceutical industry, who are also member of the orphan drug working group, were interviewed. They were selected according to their familiarity with the SSF.

The international part of the study aimed to find comparable systems to the SSF in France, the Netherlands and Spain. Different information sources were searched. Additionally, a selection of typical SSF cases was used as a way to describe how they are handled in the studied countries.

8.1 ELIGIBILITY CRITERIA FOR SSF INTERVENTION

The SSF administrative data sample did contain sufficiently detailed information to assess the interpretation of the criteria by the SSF. Indeed, for rejected applications, only the distinction "reimbursement of medical costs provided in Belgium" or "abroad" is available. The following results are mainly derived from the interviews with the members of the college of medical directors.

8.1.1 Expensive

The SSF regulation stipulates the medical treatment (or technique or drug) has to be expensive. The minimum amount of expenses however is not regulated except for the additional costs for chronically ill children. In current practice the SSF uses its own criteria. The patient's socio-economic status is not taken into account when defining the notion "expensive".

As the SSF has a closed budget, the SSF may limit the reimbursement of the costs to a percentage of the total cost. Mostly a percentage of 60% to 75% is granted. If the SSF reimbursed up to 75% the personal share of the patient is limited to an amount varying between €1000 and €1500 annually.

In some specific cases such as for instance, if there was no agreement on the price of a drug (e.g. Flolan) between the FOD Economie, KMO, Middenstand en Energie/ SPF Economie, P.M.E., Classes moyennes et Energie and the pharmaceutical firm or in case of off label use of drugs, the SSF can grant a reimbursement limited to 60%. In case the SSF grants 60% of reimbursement, there is no maximum annual personal share.

It is possible that the SSF financed an additional part after the final price setting. This provisional limitation causes doubt on final acceptance of the global cost for the patients. It results in difficulties for the prescribing doctor on having the potential risk accepted by the hospital (that will advance the costs) and complicates unnecessarily the follow-up by the providing (pharmaceutical) company.

8.1.2 Proven scientific value and prescription by a recognised specialist in the respective domain

Today the required level of scientific value is different depending on whether the medical costs relates to a rare disease or a rare indication. Whereas for rare indications the intervention has to have scientific value and effectiveness recognized by medical authorities and needs to have outgrown the experimental stage, this requirement is not set for the rare diseases. Interventions for rare diseases need to be indicated by medical authorities in the domain as the specific physiopathological approach for the respective rare disease. The underlying reason for this different approach is not clear and seems to lack any logic.

The proof of scientific value by means of classical studies for treatment or drugs for rare diseases or indications is often problematic since the disease is too rare (and the patient group too small) to have these studies performed. According to some of the physicians and representatives of the pharmaceutical industry it is obvious that the SSF should not be used for financing clinical studies, but the fact a clinical study is ongoing, may not automatically result in a non-acceptance of reimbursement of costs for patients that cannot be included in the study (eventually not meeting eligibility criteria or for other indications than these that are object of the clinical study).

8.1.3 Threatening the vital functions of the patient

In the preparatory documents of the law of 2005 the Minister explicited that a vital function is "an essential function such as the sight, even if the disease the patient is suffering from is not life threatening". A point of discussion is if the concept "vital" only refers to a primordial interest for living or also for functioning, allowing psychosocial diseases and quality of life to be taken into account. It was not possible to derive the interpretation of this notion from the sample.

8.1.4 Rare indication/disease

According to the interviewed medical directors of the College, the rareness of a disease is regarded in perspective to the orphaned criterion of a prevalence of 1/2000. This criterion is not included in legislation.

8.2 INFORMATION CHANNELS ON THE EXISTENCE OF THE SSF

As mentioned before, it was impossible to assess whether there is a lack of knowledge on the existence of the SSF among the potential target group of the SSF. Most of the interviewed stakeholders perceive the existence of the SSF as poorly known by the parties (potentially) concerned. The interviewed stakeholders are informed on the existence of the SSF through diverse channels. In 2007 the SSF sent information brochures to the patients, the hospitals, the specialists, the pharmacists and the patient organizations. We observed, however, that the SSF brochure or information initiatives by the SSF itself were hardly mentioned by the interviewed stakeholders.

8.3 APPRAISAL BY STAKEHOLDERS OF THE APPLICATION PROCEDURE AND DECISION-MAKING PROCESS

8.3.1 Clarity of the eligibility criteria

There is consensus between the interviewees that the legal criteria and requirements for eligibility are open for interpretation. Particularly, "expensive treatment", "rare disease", "experimental phase" and "vital functions" are terms that might cause confusion and discussion. Some respondents consider the broadness and vagueness of the criteria as an obstacle for coherent decision-making. However, most of them welcome the possibility of interpretation, given the role of the SSF as an additional safety net.

8.3.2 Duration

The duration of the procedure (throughput time from initiation to decision) is judged by many respondents as problematic. Yet, the time aspect is of an utmost importance since patients often wait for the decision to start the treatment. The bottleneck is seen at the level of the sickness funds since an application, once it is send to the SSF, is quickly decided upon at SSF level. Indeed, results from the sample data analysis indicate that today about 90% of the cases are handled at the SSF level within one month. It was impossible however to quantify the overall throughput time because the available data solely concern the assessment at the SSF. There were no data available on the reason(s) for the probable bottleneck in the pre-trajectory of the SSF. Yet it is reasonable to assume that passing through the different stages implies a repetitive administrative burden at each level and paves the way to delays. Indeed, the administrative check on the completeness of the file as well as a medical assessment happens at each level. Other possible underlying clarifying factors are: the non timely introduction of the required documents by the patient to the local sickness fund, delays at the financial service level of the hospital responsible for delivering the invoice, etc…

Another problematic issue is the lack of a rapid procedure for individuals in urgent need of a particular medical treatment, device or drug. Moreover renewals follow the same procedure as new applications.

Although there is no strict criterion for the delegation to one member of the SSF, the data analysis on the SSF sample reveals that decisions on renewals are to an increasing extent delegated to the leading medical officer of the SSF.

8.3.3 Administrative burden

The administrative burden is reported to be primarily situated at the level of the social service of the hospital. There is consensus between the interviewees that the administrative burden is heavy. The following examples illustrate this statement:

- Administrative formalities for renewals although it is clear the patient will need the treatment for a long period;
- Providing the same information for comparable cases the SSF already possesses resulting in duplication of information (and workload);
- Provision of information which is already at the disposal of the SSF as part of the NIHDI (financial costs of material, drugs, …);
- Obligation to provide the hospital's invoice for the material or the drug when entering a SSF file. This is a heavy administrative workload and can easily be postponed till after a positive decision on the application. For the financial department the search of invoices (invoice to the hospital) is rather complicated since drugs and medical devices are not bought piece by piece and invoices contain various products;
- Non acceptance of an electronic signature on the medical file entered by the prescribing medical doctor.

At patient level the provision of the declaration on honor that all other legal reimbursement sources in Belgium as well as abroad, or reimbursement by private insurance have been exhausted can cause problems. Our research showed that this is a purely administrative item and that the SSF is not able to check the existence of possible individual rights to reimbursement or if a patient has already (eventually partially) been compensated through other channels. However it can cause a delay on the decision if it is missing. It might result in a non reimbursement if the patient died in the meantime.

8.3.4 Reporting and transparency

For decisions taken by 'one member of the College' only negative decisions are also signed by a second member of the college (medical director of the sickness fund of the patient). No further reporting is being organized. For delegated decisions to the local sickness funds, the only reporting is financial via the NIHDI.

All stakeholders clearly stress the unclearness of (the interpretation of) several criteria. The motivation of the decisions is perceived as administrative since the legal criteria on which the decision was based are mentioned.

Currently legislation provides that an annual report has to be presented to the Verzekeringscomité/Comité de l'assurance and the Algemene Raad/Conseil Général. There is no legal obligation to make these annual reports publicly available.

8.3.5 Stakeholder involvement

The SSF is perceived as very distant and totally absent from the healthcare scene. No contacts exist with patient groups, the medical profession or the hospital services. Such contacts are reported to be avoided and even refused. Dissemination of the decision to the respective social service the patient contacted or the treating physicians is absent. Yet, this information is conceived as necessary by these stakeholders. Individual decisions on applications are not transferred to the providers of drugs or devices subject to the SSF application (delivering medical companies). As such this is acceptable and obvious because of privacy issues.

In principle it is possible to contact the SSF; the phone number and the email address are mentioned at the NIHDI website.

8.3.6 Sufficient expertise at SSF level

The interviewed physicians in particular question if the expertise at the level of the College of medical directors is sufficient. The variety and rareness of the diseases/indications makes the assessment by the same panel of "experts" extremely difficult. One cannot expect them to have all necessary knowledge on all cases submitted. Although the possibility of consulting external experts by the SSF College exists, until today this is rarely used. There is however representation of the Drug Reimbursement Commission in the College of medical directors.

The SSF leading medical officer stressed that the quality of prescriptions, the medical files and the evidence is often insufficient. This hampers a proper assessment of the case at stake by the members of the College.

8.3.7 Appeal

If the patient disagrees with the decisions of the SSF, he/she can launch an appeal to the competent labour court. Patient organisations state that most patients are not aware of the appeal procedure or judge it as heavy. The judgments of the labour court are not systematically monitored by the SSF and do not serve as guidelines for future decisions on individual cases. Similar cases are not reconsidered or reviewed by the SSF. This is not so surprising since there is no continuity and uniformity in the interpretation of some of the eligibility criteria by the courts. A fundamental criticism is that the expertise at court level is often not sufficient to judge on the mostly very specific medical issues. One could envisage installing a proper appeal procedure at SSF or NIHDI level, although legally all disputes regarding the legislation on the compulsory health care insurance fall within the competence of the labour courts. Installing such an appeal procedure does not completely solve the issue of inadequate expertise because of the variety of medical domains. External experts could however be implied.

8.4 EXPLORATION OF (COMPARABLE) SAFETY NETS IN A SELECTION OF FOREIGN COUNTRIES

No mirror system comparable to the SSF was found in the studied countries. There are however interesting mechanisms aiming at the management of rare diseases and the early access to new drugs. A comparison of the reimbursement of a selection of products reimbursed by the SSF reveals that some of these products are not reimbursed in the studies countries.

8.4.1 Early access system to new drugs

The notion of "new drug" is considered as new for a particular indication which implies that off label use is also considered. Early access to new drugs is most often during the third phase of the clinical trial and when its safety and efficacy are strongly assumed. France and Spain have implemented an early access system. In France, temporary authorisations (ATU) allows the use of drugs, outside of the framework of clinical trials, without Marketing authorisation (MA) in France, whether they benefit or not from a MA abroad. Solely drugs that are used for the treatment of serious or rare diseases, for which there is no other treatment available and cost-benefit analysis of the treatment was conducted, with a positive result are eligible for ATU. Off-label use of drugs is not eligible for reimbursement. The authorisation can be granted by AFSSAPS for use at individual patient level (at the request of the prescribing physician) or homogeneous group (cohort) level (at the request of the pharmaceutical firm) in a very short time laps. For cohort ATU a temporary use authorisation must be accompanied by a simultaneous demand for a MA or an intention to file an MA dossier in the near future. Moreover cohort ATU is subordinated to the implementation of a protocol for therapeutic use and information collection. For nominative ATU, the agency notably relies on a dossier on quality, efficacy and safety of the medicinal product supplied by the pharmaceutical company at its request and, if necessary, by the ATU applicant. In France, drugs used at hospital level are integrated in the hospital budget.

These budgets are complemented if the hospitals treat patients that need expensive (or orphan) drugs. Drugs for outpatients are reimbursed at 100% by Sickness Insurance on the basis of their transfer price (prix de cession). This price comprises the purchase price (based on price convention between the hospital and the pharmaceutical laboratory) + lump sum margin for the costs of administration and distribution by the hospital + VAT.

Belgium does not have a specific "early access to new drugs" system for patient groups paid by public means. Early access to drugs for individual patients is possible via the SSF (patient initiative). Early access to drugs for groups of patients is solely possible in within the scope of compassionate use (for drugs which do not yet have obtained a MA in Belgium) and medical need programs (which concerns medicinal products which have a MA in Belgium for a given indication, but are used for another indication). These programs are set-up and paid by the pharmaceutical company, and the medicines are provided for free.

In France, however, the access to drugs without MA in France for groups of patients can be financed with public means via the ATU procedure.

8.4.2 Centralisation of the treatment of rare diseases in reference centres

Rare diseases are complex and demand high expertise. In France as well as in Spain and the Netherlands, knowledge and patient treatment is clustered in reference centres (or specific hospitals). Costs of treatments and drugs are included in the reference centers' budgets. The results from the SSF sample reveal that a small number of hospitals present a large number of applications, which implies that "de facto" there is a kind of reference centre system for rare diseases in Belgium. There is however no regulation with regard to the systematic financing of the treatment in those particular hospitals.

At the European level, the High Level Group on Health Services and Medical Care developed some principles regarding European reference networks for rare diseases, including their role in tackling rare diseases and other conditions requiring specialized care, patient volumes and some criteria that such centers should fulfill. European reference networks should also serve as research and knowledge centers, updating and contributing to the latest scientific findings, treating patients from other Member States and ensuring the availability of subsequent treatment facilities where necessary.

8.5 OPTIONS FOR OPTIMISATION

When making the round up of the elements allowing an overall evaluation of the functioning of the SSF, one cannot pass up on the theoretical reflection on the unmet needs in a broader scope than the current legal framework. Starting from the actual structure and operation of the SSF, possible alternatives and options to optimize the management of high-cost, yet medically necessary care that is not reimbursed by the compulsory health insurance are discussed. The focus of this chapter however is not the implementation of possible alternatives, as these will mostly need further research. Consequently we will not zoom into any feasibility constraints. Although this section is a rather theoretical reflection and to this extent does not necessarily build on the results presented in previous chapters, it has to be noted that some of the alternatives were spontaneously mentioned by the stakeholders during the interviews.

8.5.1 Patient initiative (SSF) versus automatic entitlement (compulsory health care insurance)

The SSF system implies the initiative of the patient, supported by his treating medical doctor, submitting an application for reimbursement without an a priori guarantee of actual reimbursement. Such an approach has the major disadvantage that not all patients potentially eligible to benefit from reimbursement will be reached. This might be due to various reasons such as a lack of information on the existence of the SSF, insufficient comprehension of the eligibility criteria, etc. Individuals entitled to the compulsory health care insurance on the other hand have an automatic right to the services that are covered in the nationally established fee schedule (the so-called nomenclature). Sickness funds are legally bound to reimburse any claim from their insured members for care delivered by any recognized health care provider at the agreed fee levels.

Hence it is primordial to (re)consider if the categories currently represented in the SSF system can be introduced into the benefit package of the compulsory health care insurance. A precondition for a similar shift is that realistic budget estimates can be made and that reimbursable items can be defined in advance. Each item needs a new (pseudo)nomenclature number.

8.5.2 Revision of the Categories

8.5.2.1 Reimbursement of extra medical costs for chronically ill children: shift back to the compulsory health care insurance?

Although there are arguments to consider the group of chronically ill children as a specific target population for the SSF, it can be questioned whether the differentiation according to age groups does not create a discriminatory situation Moreover the limitation of this category to children can be problematic since reimbursement of cost for the treatment of chronically ill children would abruptly stop once they become adults (>19 years old). As soon as the individual is adult, a maximum annual personal share will be calculated. The costs for Epidermolysis Bullosa however are reimbursed in full, irrespective of individual's age.

8.5.2.2 Reimbursement of expenses for medical treatment abroad: suppress the discrimination?

The SSF refunds costs for non-reimbursable medical treatment abroad, as well as travel and accommodation expenses for the patient and for his/her companion, if "worthy of consideration". The SSF itself indicates in the annual report 2007 that reimbursed costs in this category mainly include travel costs to neighboring countries. This may lead to a possible discrimination of the patients treated in Belgium for whom the costs of accommodation or the travel cost are not reimbursed although the distance between the domicile and the respective hospital approaches the distance to a hospital in a neighboring country.

8.5.2.3 *Innovative medical implants and techniques: interaction with the category 5 of art. 35 § 3*

The SSF procedure for this category has some points in common with category 5 of art. 35 § 3 of the Nomenclature which provides a conditional reimbursement of implants and invasive devices with no proven clinical effectiveness and safety. The major difference with the category 5 regulation is that the SFF procedure provides a limited timeframe of reimbursement of 2 years and that reimbursement concerns individual cases. It is unclear however what research design is used to evaluate the respective implants. It has to be noted that the current application of the category 5 regulation also rarely involves a research design needed to prove the added value of the emerging technology. This approach can lead to budgetary failure or inappropriate use of resources. Another possible negative effect is the diffusion of unevaluated medical technologies and risk of safety problems. An elaborated description of a transparent and scientifically valid procedure to evaluate medical devices early, ideally before being introduced into the market, can be found in report nr. 44A.[44] Although category 5 of art. 35 only deals with implantable devices, the procedure (or a similar one) can also be applied to other medical devices.

8.5.2.4 *From rare disease or rare indication to expensive medical care due to justified medical treatment?*

The category "rare disease and rare indications" limits the action field of the SSF considerably. Given the SSF's role as a subsidiary safety net, the focus of the SSF could be broadened to expensive medical costs due to a medical treatment that is judged as appropriate, regardless of the question whether the disease or the indication is rare or not.

It should be noted however that some reflection on the notion of "justified medical treatment" is necessary in case of implementation. It is obvious that misuse of the SSF by pharmaceutical firms derogating from the regular circuit has to be avoided. If there is no MA and/or reimbursement by the obligatory health care insurance for interventions, drugs or implants for frequently occurring diseases or indications, this will often be due to a weak evidence base or a disbelieve in the effectiveness by the respective firm (Indeed, for more frequently occurring diseases, it is -relatively- easier to obtain evidence in a shorter timeframe). One should avoid that patients are exposed to insufficiently evaluated technologies

8.5.3 Alternative system: a franchise system for high medical expenses due to a justified medical condition or treatment

Although falling outside the initial scope of this study, it is worthwhile to consider the SSF from the perspective of the health insurance system as a whole in its societal context. It is clear that the SSF is a residual system, aimed at avoiding people to incur catastrophic expenses due to medically justified healthcare. Whether or not the level of expense beyond which it is to be considered as catastrophic should be modulated by the income of the patient or his household, remains debatable. If the SSF is indeed considered as a residual safety net for justified healthcare expenses currently not covered, for one reason or another, by the compulsory health insurance, it can be questioned why it should be limited to people with e.g. a rare disease or indication. An alternative system could be a franchise system for high medical expenses due to a justified medical condition or treatment. To be sustainable, a deductible (franchise) could be set. It could be envisaged to lower the deductible whenever high expenses are still occurring during several consecutive year, so as to avoid impoverishment through a chronic condition.

Besides the limitations set by the eligibility criteria, the following costs should continue to be excluded from the system:

- Supplements. A point of discussion however are the supplements linked to treatment of chronically ill children that are currently eligible for reimbursement by the SSF;
- Costs already borne by other insurances or reimbursement systems.

Switching to a franchise system does not imply that the currently existing case by case handling discontinues to exist or would be radically changed, since a proper assessment of the eligibility criteria remains necessary. Transitional measures for patients passing from one system to another should be provided.

8.5.4 Coherent Drug reimbursement policy

Diseases, indications or medical situations that are currently not (yet) covered by the compulsory health care insurance, risk to fall as a residual under the SSF umbrella. Hence, the SSF is often considered as a temporary solution ('waiting room') for specific or complex situations for which a framework for reimbursement has not been established yet. This is especially the case for drugs, as they have been responsible for the major part of the SSF expenses during the last years. As of today, drugs are often reimbursed by the SSF during several years, with no real assessment nor a formal price setting occurring in the meantime. Pharmaceutical companies are indeed not always eager to introduce a request for reimbursement by the compulsory insurance system at the Drug Reimbursement Commission (DRC; Commissie Tegemoetkomingen Geneesmiddelen/Commission Remboursement des medicaments), because such a request is a costly procedure. Commercial or financial arguments – in particular when dealing with drugs for rare diseases with a small potential market - often seem to restrain pharmaceutical companies from starting up this process. Moreover, in such cases effectiveness is difficult to prove since the number of patients is inevitably small.

Entering an application for reimbursement of a drug (or expanding its indications) in the compulsory health system also implies a discussion on price setting. The fact that pharmaceuticals are reimbursed by the SSF for a very long period can have as an effect that this discussion is postponed indefinitely. This may result in a discrimination against patients with a disease or indication currently falling outside the scope of the SSF, whereas patients with a rare disease or indication are to a large extent reimbursed by the SSF.

Such a misuse results in the unjustified use of public means. One way to counter the misuse of the SSF as a waiting room and/or the "bypass" of the regular system, would be to request that the pharmaceutical firm submits an MA application (if not yet the case) and commit itself to introduce a demand at the DRC.

On the other hand the SSF is regularly 'misused' or bypassed if the price the pharmaceutical firm proposes for the drug was not accepted (for instance if the DRC judged a priori that the proposed price is too high and does not assess the evidence). In that case, the sole solution for the patient to get reimbursement of the product at stake is the SSF. Systematic collaboration between the SSF and the different interfering bodies regarding reimbursement policy and marketing authorization of drugs and implants, such as for instance the DRC, the College van geneesheren voor weesgeneesmiddelen (CMDOD) and the Federal Agency for Medicines and Health Products is therefore necessary to avoid contradictory decisions and to centralize expertise.

9 APPENDICES

9.1 SSF LEGISLATION

Afdeling VII

Bijzonder solidariteitsfonds

[**I** - Wet 27-4-05 - B.S. 20-5 – ed. 2]

Onderafdeling I.

Algemeenheden]

Art. 25. Bij de Dienst voor geneeskundige verzorging wordt een Bijzonder solidariteitsfonds opgericht, dat wordt gefinancierd door een voorafname op de in artikel 191 bedoelde inkomsten, waarvan het bedrag voor ieder kalenderjaar wordt vastgesteld door de Koning bij een besluit vastgesteld na overleg in de Ministerraad.

Het College van geneesheren-directeurs beslist binnen de perken van de financiële middelen van dit Fonds over de tegemoetkomingen vanuit dit Fonds aan de in de artikelen 32 en 33 bedoelde rechthebbenden.

Het Bijzonder Solidariteitsfonds verleent slechts een tegemoetkoming indien is voldaan aan de in deze afdeling gestelde voorwaarden en indien de rechthebbenden hun rechten hebben doen gelden krachtens de Belgische, buitenlandse of supranationale wetgeving of krachtens een individueel of collectief gesloten overeenkomst. Het Fonds verleent slechts tegemoetkomingen in de kosten van geneeskundige verstrekkingen waarvoor, in het concrete geval, in geen tegemoetkoming voorzien is krachtens de reglementaire bepalingen van de Belgische verzekering voor geneeskundige verzorging of krachtens de wettelijke bepalingen van een buitenlandse regeling voor verplichte verzekering.

Worden niet ten laste genomen door het Bijzonder Solidariteitsfonds:

1° De persoonlijke aandelen bedoeld in de artikelen 37 en 37 bis en de supplementen op in toepassing van de reglementering van de verplichte verzekering voor geneeskundige verzorging vastgelegde prijzen en honoraria;

2° De supplementen bedoeld in artikel 90 van de wet op de ziekenhuizen, gecoördineerd op 7 augustus 1987, en de comfortkosten.

Onderafdeling II.

Tegemoetkoming voor zorgen verleend in België

Art. 25bis. In het kader van de in België verstrekte verzorging kan het College van geneesheren-directeurs tegemoetkomingen verlenen in de kosten van de geneeskundige verstrekkingen in zeldzame indicaties.

Deze verstrekkingen moeten bovendien voldoen aan elk van de volgende voorwaarden:

a) de verstrekking is duur;

b) de verstrekking bezit een wetenschappelijke waarde en een doeltreffendheid die door de gezaghebbende medische instanties in ruime mate worden erkend en het experimenteel stadium is voorbij;

c) de verstrekking wordt gebruikt voor de behandeling van een aandoening die de vitale functies van de rechthebbende bedreigt;

d) er bestaat geen alternatief op medisch-sociaal vlak inzake diagnose of therapie in het kader van de verplichte verzekering voor geneeskundige verzorging;

e) de verstrekkingen worden voorgeschreven door een geneesheer-specialist, gespecialiseerd in de behandeling van de betreffende aandoening, en die gemachtigd is om de geneeskunde uit te oefenen in België.

Het feit dat de gevraagde verstrekking niet terugbetaald wordt in het kader van de verplichte verzekering voor geneeskundige verzorging of ten minste niet voor de indicatie die de aanvraag rechtvaardigt, terwijl dit wel het geval zou kunnen zijn voor andere indicaties, vormt op zich geen aanduiding voor het zeldzame karakter ervan.

Art. 25ter. § 1. Het College van geneesheren-directeurs kan aan rechthebbenden die lijden aan een zeldzame aandoening tegemoetkoming verlenen in de kosten van de geneeskundige verstrekkingen.

Deze verstrekking moet voldoen aan elk van de volgende voorwaarden:

a) de verstrekking is duur;

b) de verstrekking wordt door de gezaghebbende medische instanties op gemotiveerde wijze aangeduid als de specifieke fysiopathologische aanpak van de zeldzame aandoening;

c) de verstrekking wordt gebruikt voor de behandeling van een aandoening die de vitale functies van de rechthebbende bedreigt;

d) er bestaat geen therapeutisch alternatief in het kader van de verplichte verzekering voor geneeskundige verzorging;

e) de verstrekkingen worden voorgeschreven door een geneesheer-specialist, gespecialiseerd in de behandeling van de betreffende aandoening, en die gemachtigd is om de geneeskunde uit te oefenen in België.

In het kader van het Bijzonder Solidariteitsfonds kan er niet worden afgeweken van de beslissingen van het College van geneesheren voor weesgeneesmiddelen, bedoeld in artikel 2 van het koninklijk besluit van 8 juli 2004 betreffende de vergoeding van weesgeneesmiddelen.

§ 2. Het College van geneesheren-directeurs kan aan rechthebbenden die lijden aan een zeldzame aandoening die een continue en complexe verzorging vereist, een tegemoetkoming verlenen in de kosten ervan.

Een tegemoetkoming in de kostprijs van deze verzorging kan worden verleend, indien wordt voldaan aan elk van de volgende voorwaarden:

a) de verzorging is in haar totaliteit duur;

b) de verzorging heeft betrekking op een bedreiging van de vitale functies van betrokkene die een rechtstreeks en specifiek gevolg is van de zeldzame aandoening;

c) er bestaat geen therapeutisch alternatief in het kader van de verplichte verzekering voor geneeskundige verzorging;

d) de complexe verzorging worden voorgeschreven in het raam van een behandelingsplan door een geneesheer-specialist gespecialiseerd in de behandeling van betreffende aandoening en die gemachtigd is om de geneeskunde uit te oefenen in België.

Het College van geneesheren-directeurs stelt de verstrekkingen vast die voldoen aan bovenbedoelde voorwaarden.

Art. 25quater Het College van geneesheren-directeurs kan gedurende een bepaald tijdvak tegemoetkomingen verlenen in de kosten van medische hulpmiddelen en verstrekkingen, met uitzondering van de geneesmiddelen, die innovatieve medische technieken zijn.

Deze medische hulpmiddelen en verstrekkingen moeten voldoen aan elk van de volgende voorwaarden:

a) de medische hulpmiddelen en verstrekkingen zijn duur;

b) de medische hulpmiddelen en verstrekkingen zijn zeldzaam;

c) de medische hulpmiddelen en verstrekkingen worden door gezaghebbende medische instanties op gemotiveerde wijze aangeduid als de aangewezen wijze voor het behandelen van een bedreiging van de vitale functies van de rechthebbende en zijn het experimentele stadium voorbij;

d) de verstrekkingen bezitten na een kosten/baten afweging een belangrijke en aangetoonde meerwaarde;

e) de verstrekkingen worden voorgeschreven door een geneesheer-specialist, gespecialiseerd in de behandeling van de betreffende aandoening, en die gemachtigd is om de geneeskunde uit te oefenen in België;

f) bij de bevoegde technische Raad werd een aanvraag ingediend om de medische meerwaarde te evalueren en/of de tegemoetkoming van de verplichte verzekering in de kosten van deze verstrekkingen te verkrijgen.

Op voorstel van het College van geneesheren-directeurs, legt het Verzekeringscomité de limitatieve lijst aan van medische hulpmiddelen en verstrekkingen, bedoeld in het eerste lid, waarvoor gedurende een beperkt tijdvak van ten hoogste één jaar aldus een tegemoetkoming kan worden verleend. Dat tijdvak kan éénmaal worden vernieuwd en het Comité dient deze beslissing te motiveren.

(°°) De bepalingen van dit artikel zijn van toepassing voor de verstrekkingen die zijn verleend vanaf 1 april 2005

Art. 25quinquies. § 1. Het College van geneesheren-directeurs kent, onder de in dit artikel bepaalde voorwaarden, de tenlasteneming toe van het geheel van de bijkomende kosten die samenhangen met de medische behandeling van chronisch zieke kinderen die jonger zijn dan 19 jaar, en dit vanaf het ogenblik dat deze kosten een bedrag bereiken van 650 euro.

Deze tegemoetkoming blijft verschuldigd voor alle kalenderjaren gedurende welke de bijkomende kosten een bedrag van 650 euro bereiken, alsmede voor het eerste daaropvolgende kalenderjaar gedurende hetwelk dit bedrag niet werd bereikt.

Deze tegemoetkoming wordt opnieuw verworven voor het kalenderjaar gedurende hetwelk de bijkomende kosten die in aanmerking worden genomen, 650 euro bereiken.

Onder chronisch ziek kind wordt een kind verstaan dat lijdt aan één van de volgende aandoeningen:

- kanker;

- nierinsufficiëntie in chronische behandeling via peritoneale of hemodialyse;

- een andere levensbedreigende ziekte die een continue behandeling van minstens zes maanden noodzaakt of een repetitieve behandeling met dezelfde duur.

§ 2. De extra-kosten hebben betrekking op geneeskundige verstrekkingen die voldoen aan elk van de volgende voorwaarden:
a) de verstrekkingen hebben een wetenschappelijke waarde en een oeltreffendheid die door de gezaghebbende medische instanties in ruime mate wordt erkend;

b) er bestaat geen aanvaardbaar alternatief voor, vanuit medisch-sociaal oogpunt, op vlak van therapie of preventie in het kader van de verplichte verzekering voor geneeskundige verzorging;

c) de verstrekkingen zijn voorgeschreven door een geneesheer-specialist gespecialiseerd in de behandeling van betreffende aandoening, die de behandeling coördineert of door de behandelende arts indien de verstrekkingen zijn hernomen op het behandelingsplan bedoeld in § 3, en die gemachtigd is om de geneeskunde uit te oefenen in België.

§ 3 De verstrekkingen welke extra-kosten teweeg brengen worden door de in § 2, c) bedoelde geneesheer-specialist voorgeschreven in het raam van een behandelingsplan.

Het College van geneesheren-directeurs stelt de verstrekkingen vast die voldoen aan bovenbedoelde voorwaarden.

§ 4. In afwijking van artikel 25, vierde lid, kan als bijkomende kost worden aangemerkt, het persoonlijk aandeel dat niet in aanmerking wordt genomen in de optelling van de remgelden uitgevoerd in het raam van de maximumfactuur, evenals [...] de veiligheidsmarge, bedoeld in artikel 35, § 4, 2° en 3° en in artikel 35bis van de bijlage bij het koninklijk besluit van 14 september 1984 tot vaststelling van de nomenclatuur van de geneeskundige verstrekkingen inzake verplichte verzekering voor geneeskundige verzorging en uitkering.

Onderafdeling III.
Tegemoetkoming in het raam van in het buitenland verleende verzorging

Art. 25sexies. Het Bijzonder Solidariteitsfonds kan in behartigenswaardige gevallen de medische kosten van de rechthebbenden ten laste nemen voor in het buitenland verleende geneeskundige verstrekkingen, waarvoor toestemming werd verleend door de adviserend geneesheer overeenkomstig de vigerende Belgische, internationale of supranationale wetgeving, alsook de gerelateerde reis- en verblijfskosten van de rechthebbende en, in voorkomend geval, van de persoon die hem vergezelt voor bedoelde verstrekkingen.

De geneeskundige verstrekkingen, verstrekt in het buitenland, moeten worden voorgeschreven vooraleer de zorgen worden toegediend door een geneesheerspecialist, gespecialiseerd in de behandeling van betreffende aandoening en die gemachtigd is om de geneeskunde uit te oefenen in België.

Onderafdeling IV.
Procedures

Art. 25septies. § 1. De Koning kan, na advies van het Verzekeringscomité, de procedures van aanvraag, en toekenning van de tegemoetkoming vaststellen.

De aanvraag om tegemoetkoming van de verzekering in de kosten van de verstrekkingen, bedoeld in de onderafdelingen II en III, wordt door de rechthebbende van de verzekering voor geneeskundige verzorging bij de adviserend geneesheer van zijn verzekeringsinstelling ingediend bij een ter post aangetekende brief of op gelijk welke andere manier die toelaat de datum van indiening met zekerheid vast te stellen.

De rechthebbende is evenwel niet verplicht een aanvraag in te dienen voor tegemoetkoming in de extra kosten, bedoeld in artikel 25quinquies. Voor deze extra kosten kan de verzekeringsinstelling waarbij de betrokkene is ingeschreven of aangesloten, alsmede de andere partijen die tussenkomen in de tenlasteneming zelf een aanvraag indienen, op basis van de elementen waarover zij beschikken en na akkoord van de betrokkene.

(°°) De bepalingen van dit artikel zijn van toepassing voor de verstrekkingen die zijn verleend vanaf 1 april 2005

(°) De bepalingen van dit artikel zijn van toepassing voor de verstrekkingen die zijn verleend vanaf 1 april 2005

De aanvraag om tegemoetkoming moet minstens, bevatten:

1° een inlichtingsblad waarvan het model wordt opgemaakt door het Verzekeringscomité, op voorstel van het College van geneesheren-directeurs en dat door de adviserend geneesheer van de verzekeringsinstelling wordt voorgelegd aan het College van geneesherendirecteurs, binnen een termijn van dertig dagen vanaf de dag van de door de rechthebbende ingediende aanvraag. Elke vraag om bijkomende informatie, rechtstreeks geadresseerd aan de rechthebbende, schort de termijn van dertig dagen op. Dit geldt op dezelfde wijze wanneer deze rechthebbende in kennis wordt gesteld van het feit dat bijkomende informatie werd gevraagd;

2° een voorschrift, waarvan het model, op voorstel van het College van geneesheren-directeurs, kan worden vastgesteld door het Verzekeringscomité, en dat door een geneesheer wordt opgemaakt, en waarbij een omstandig geneeskundig verslag is gevoegd dat alle inlichtingen bevat die toelaten te besluiten of de gevraagde verstrekking voldoet aan de voorwaarden die vermeld zijn in de onderafdelingen II en III.

3° een gedetailleerde factuur of een omstandig bestek ingeval van een principiële aanvraag met de kosten, opgemaakt door de zorgverlener(s);

4° de verklaring op erewoord, waarvan het model wordt opgemaakt door het Verzekeringscomité op voorstel van het College van geneesheren-directeurs waarin de rechthebbende:

- bevestigt dat hij, in verband met de verstrekkingen waarvoor hij een tegemoetkoming vraagt, zijn rechten heeft uitgeput krachtens de Belgische of buitenlandse wetgeving en geen rechten kan doen gelden krachtens een individueel of collectief gesloten overeenkomst;

- meedeelt ten belope van welk bedrag hij, in voorkomend geval, rechten kan doen gelden krachtens de voornoemde overeenkomst;

- bepaalt of hij de door de verplichte verzekering voor geneeskundige verzorging toegestane vergoedingen in het raam van het Bijzonder Solidariteitsfonds, al of niet zelf zal innen.

§ 2. De in artikel 153 bedoelde adviserend geneesheer stuurt de aanvraag niet naar het College van geneesheren-directeurs door indien ze betrekking heeft op:

- huisvestings-, verblijfs- of reiskosten van de rechthebbende of zijn vergezellend persoon gedurende de opneming in een Belgisch ziekenhuis;

- supplementen of persoonlijke aandelen voor verstrekkingen die werden verleend overeenkomstig gelijk welke regeling die de voorwaarden omvat voor de tegemoetkoming van de verzekering voor geneeskundige verzorging voor bepaalde verstrekkingen inclusief de in het buitenland verleende verzorging. Wanneer de aanvraag echter kan worden onderzocht in het kader van artikel 25quinquies, dient ze te worden overgezonden aan het College van geneesheren-directeurs.

- geneeskundige verzorging en/of reis- en verblijfskosten voor een rechthebbende die in het buitenland verzorging ontvangt en waarvoor de adviserend geneesheer van de verzekeringsinstelling geen toestemming heeft verleend.

- een verstrekking verricht meer dan drie jaar voor de aanvraag.

Art. 25octies. De Koning kan de voorwaarden bepalen waaronder de beslissingsbevoegdheid van het College van geneesheren-directeurs uitgeoefend kan worden door één of meerdere geneesheren, lid van het voornoemde College. Hij stelt meer bepaald de criteria vast met betrekking tot het bedrag, de facturatie en de betaling van de tegemoetkoming die het bovenbedoelde College in aanmerking neemt wanneer hij de beslissingsbevoegdheid toekent. Deze beslissingsbevoegdheid kan in geen enkel geval exclusief uitgeoefend worden door geneesheren die tewerkgesteld zijn bij de verzekeringsinstellingen waarbij de belanghebbende rechthebbende aangesloten of ingeschreven is. In geval het College op bestek over de gevraagde tegemoetkoming van het Bijzonder Solidariteitsfonds beslist, neemt het College een principieel akkoord op basis waarvan de betrokken verzekeringsinstelling kan tegemoetkomen. In dit geval zendt de betrokken verzekeringsinstelling driemaandelijks een verzamelstaat over van de in uitvoering van de collegebeslissingen uitbetaalde bedragen.

Tijdens de bijeenkomst tijdens dewelke de aanvraag wordt behandeld, kan het College van geneesheren-directeurs inzake onder meer indicatie en/of richtprijs advies inwinnen bij de gezaghebbende-wetenschappelijke instanties, bij de officiële organen in de schoot van het RIZIV, bij de Federale Overheidsdienst Volksgezondheid, Veiligheid van de Voedselketen en Leefmilieu of bij het federaal Kenniscentrum voor de Gezondheidszorg.

Bij gebreke aan advies of adviezen binnen de twee maand na de aanvraag gericht tot een bovenbedoelde instantie door het College van geneesheren-directeurs, beslist dit over de aanvraag tot tussenkomst. Het College formuleert de adviesaanvragen met betrekking tot eenzelfde dossier, ter gelegenheid van dezelfde zitting.

Art. 25nonies. Indien het College van geneesheren-directeurs onmiddellijk over de tussenkomst beslist op basis van een factuur, betaalt de verzekeringsinstelling aan de rechthebbende of aan de zorgverlener het toegekend bedrag binnen een termijn van 15 werkdagen na ontvangst van de kennisgeving van de beslissing van het College van geneesheren-directeurs.

Indien het College een principieel akkoord uitbrengt en de adviserend geneesheer van de verzekeringsinstelling het bedrag bepaalt, toegekend volgens de regels voorzien door het College van geneesheren-directeurs na verificatie van de factuur, betaalt de verzekeringsinstelling dat bedrag aan de rechthebbende of aan de zorgverlener binnen een termijn van 20 werkdagen na ontvangst van de factuur.

Geen enkele tussenkomst van het Bijzonder Solidariteitsfonds is verschuldigd voor een bedrag dat door de rechthebbende enkel effectief verschuldigd is in geval van gunstige beslissing.

Onderafdeling V.
Activiteitenverslag

(°) De bepalingen van dit artikel zijn van toepassing voor de verstrekkingen die zijn verleend vanaf 1 april 2005

(°)De bepalingen van dit artikel zijn van toepassing voor de verstrekkingen die zijn verleend vanaf 1 april 2005

Art. 25decies. Het College van geneesheren-directeurs maakt jaarlijks ten behoeve van het Verzekeringscomité en van de Algemene Raad, een verslag op met een inventaris van de beslissingen. In dit rapport kunnen ook voorstellen of suggesties tot verbetering of aanpassing van de verzekering voor geneeskundige verzorging worden opgenomen. Deze voorstellen of suggesties worden overgezonden aan de bevoegde technische raden belast met de opmaak van voorstellen tot wijziging van de nomenclatuur bedoeld in artikel 35.

(°°) De bepalingen van dit artikel zijn van toepassing voor de verstrekkingen die zijn verleend vanaf 1 april 2005

9.2 INFORMATION LETTER PATIENTS

9.2.1 Dutch version

RIZIV
Rijksinstituut voor Ziekte- en Invaliditeitsverzekering

Uw correspondent : A.M. Van Campenhout
Tel. 02/739.76.70

Onze referte : 1410/AVC

Brussel,

Uw medewerking aan een wetenschappelijke studie over het Bijzonder solidariteitsfonds

Geachte Mevrouw, geachte Heer,

Aangezien u een dossier hebt bij het Bijzonder solidariteitsfonds[1] (BSF), kunt u meewerken aan een wetenschappelijke studie over het BSF. Met deze brief willen wij u informatie geven over die studie en over uw medewerking daaraan.

Doel van de studie?

De studie over het BSF heeft een dubbel doel:

- de doeltreffendheid van het BSF evalueren wat betreft de huidige wettelijk bepaalde opdrachten[2].
- de rol van het BSF als sociaal vangnet bekijken vanuit een internationaal perspectief.

Onder meer de selectiecriteria van de behandelingen, de kenmerken van de patiënten en van de artsen, de bekendheid van het BSF en de aanvraagprocedure en besluitvorming zullen aan bod komen.

Wie voert de studie uit?

[1] Zie de info op de website van het Rijksinstituut voor ziekte- en invaliditeitsverzekering (RIZIV): www.riziv.be > Burgers > Medische kosten > Bijzonder solidariteitsfonds

[2] Zie ook art. 25-art.25decies (Titel III, hoofdstuk I) van de wet betreffende de verplichte verzekering voor geneeskundige verzorging en uitkeringen gecoördineerd op 14 juli 1994.

...

Tervurenlaan 211 · B-1150 Brussel Tel.: 02 739 71 11 · Fax: 02 739 72 91
Openingsuren van de kantoren: van 9 tot 12 uur en van 13 tot 16 uur. Afspraak mogelijk.

Het gaat om een studie van het Federaal Kenniscentrum voor de Gezondheidszorg (KCE)[3], een onafhankelijke, parastatale instelling die wetenschappelijke studies uitvoert om de overheid bij te staan in haar beslissingen omtrent het gezondheidszorgbeleid in België.

Wie werkt aan de studie mee?

Alle partijen, betrokken bij het BSF, zullen aan de studie meewerken: het RIZIV (waar het BSF toe behoort), de ziekenfondsen, de sociale diensten van de ziekenhuizen, de patiëntenverenigingen en ook de patiënten, dus mogelijk ook uzelf.

Waaruit bestaat uw medewerking?

Een analyse van de patiëntendossiers die bij het BSF ingediend worden, is noodzakelijk om de onderzoeksvragen van de studie te beantwoorden. U werkt dus mee aan de studie als u het KCE toelaat om de gegevens in uw dossier te gebruiken.

Gevolgen voor u?

Al dan niet meewerken aan de studie heeft voor uzelf geen gevolgen:

- Er is geen invloed op de zorg die u ontvangt of de behandeling van uw dossier bij het RIZIV.
- Er zijn geen directe voordelen of nadelen verbonden aan uw medewerking. U ontvangt bv. geen vergoeding, maar u hoeft ook geen extra kosten te maken.

Is uw medewerking verplicht?

Neen. U beslist vrijwillig of uw dossier al dan niet opgenomen wordt in de studie.
Belangrijk! Wanneer u niet uitdrukkelijk weigert om mee te werken, gaan wij ervan uit dat het KCE uw gegevens mag gebruiken.

Hoe uw medewerking weigeren?
Uw medewerking weigeren kunt u enkel als volgt:

1. Vul bijgaand weigeringsformulier in: naam + datum (zie bijlage 1).
2. Onderteken het formulier.
3. Stuur het formulier naar:

RIZIV - Dienst geneeskundige verzorging -Medische directie
TAV Van Campenhout AM
Tervurenlaan 211
1150 BRUSSEL

vóór 30-04-2009.

U kunt daarvoor gebruik maken van de bijgevoegde gefrankeerde enveloppe.
Met weigeringsformulieren die na 30-04-2009 toekomen, wordt geen rekening meer gehouden.

Vertrouwelijkheid

Alle gegevens die mogelijk van u verzameld worden in het kader van deze studie krijgen een vertrouwelijke behandeling. De resultaten van de analyse van de dossiers zullen verwerkt worden in een rapport van het KCE. De identiteit van de deelnemers zal echter op geen enkele manier uit dat rapport achterhaald kunnen worden en de onderzoekers van het KCE zullen die ook niet kennen.
Voor meer info in verband met de vertrouwelijkheid van de studie: zie bijlage 2.

Contactpersoon

Als u meer informatie wenst of vragen hebt over deze studie kunt u contact opnemen met Imgard Vinck van het KCE, die het project leidt.
Telefoon: 02/2873338

De leidend ambtenaar
van de Dienst geneeskundige verzorging van het RIZIV,

H. DE RIDDER
Directeur-generaal.

Bijlagen:
1) Weigeringsformulier
2) Info over de vertrouwelijkheid van de studie

Bijlage 1: Weigeringsformulier studie over het BSF

Ik bevestig dat ik de informatie over de studie "Bijzonder solidariteitsfonds" van het KCE heb gelezen en begrepen. Ik ga **niet akkoord** om mee te werken aan deze studie.

_____ _____

Datum Naam deelnemer of wettelijke vertegenwoordiger

_____

Handtekening deelnemer of wettelijke vertegenwoordiger

Stuur dit formulier **vóór 30-04-2009** terug naar
RIZIV - Dienst geneeskundige verzorging -Medische directie
TAV Van Campenhout AM
Tervurenlaan 211
1150 BRUSSEL

9.2.2 French version

SERVICE DES SOINS DE SANTE

Correspondant : M. Marc NOEL
Chef administratif
Tél.: 02/739 77 77 **Fax** : 02/739 78 73
E-mail:
N. réf. 1410/MN **Bruxelles,**

Votre participation à une étude scientifique sur le Fonds Spécial de solidarité

Madame, Monsieur,

Comme vous avez introduit un dossier auprès du Fonds spécial de solidarité[1] (FSS), vous pouvez participer à une étude scientifique sur le FSS. Par la présente, nous voulons vous apporter des informations sur cette étude et sur votre collaboration à celle-ci.

But de l'étude

Le but de l'étude sur le FSS est double :

- Évaluer l'efficacité du FSS en ce qui concerne la manière dont il s'acquitte de ses missions légales actuelles[2].
- Analyser le rôle du FSS comme filet de sécurité à partir d'une perspective internationale.

L'étude abordera notamment les critères de sélection des traitements, les caractéristiques des patients et des médecins, la notoriété du FSS et la procédure de demande ainsi que le processus décisionnel.

Qui réalise l'étude ?

Il s'agit d'une étude du Centre fédéral d'expertise des soins de santé (KCE)[3], un organisme d'intérêt public (parastatal) indépendant qui a pour mission de réaliser des

[1] Voir les informations sur le site web de l'Institut national d'assurance maladie-invalidité (INAMI) : www.inami.be > Assurés médicaux > Frais médicaux > Fonds spécial de solidarité

[2] Voir également art. 25-art.25decies (Titre III, chapitre I^er^) de la loi relative à l'assurance obligatoire soins de santé et indemnités, coordonnée le 14 juillet 1994.

[3] http://www.kce.fgov.be

...

Avenue de Tervueren 211 • B - 1150 Bruxelles Tél. : 02 739 71 11 • Fax : 02 739 78 73
Heures d'ouverture des bureaux : de 9 à 12 heures et de 13 à 16 heures. Possibilité de rendez-vous.

études scientifiques en vue d'aider les pouvoirs publics dans leurs décisions en matière de politique des soins de santé en Belgique.

Qui participe à l'étude ?

Toutes les parties concernées par le FSS collaboreront à l'étude : l'INAMI (dont fait partie le FSS), les mutualités, les services sociaux des hôpitaux, les associations de patients et aussi les patients, donc peut-être vous également.

En quoi consiste votre collaboration ?

Une analyse des dossiers de patient introduits auprès du FSS s'impose aux fins de répondre aux questions posées par l'étude. Vous collaborez donc à l'étude en permettant au KCE d'utiliser les données présentes dans votre dossier.

Quelles sont les conséquences pour vous ?

Le fait de participer ou non à l'étude n'entraîne aucun effet pour vous-même :

- Il n'y a aucune influence sur les soins que vous recevez ou sur le traitement de votre dossier à l'INAMI.
- Il n'y a aucun avantage ou inconvénient direct lié à votre participation à cette étude. Vous ne recevez p. ex. pas de rémunération, mais vous n'aurez pas de frais supplémentaires non plus.

Votre participation est-elle obligatoire ?

Non. Vous décidez librement de la prise en compte ou non de votre dossier dans l'étude.
Important! Si vous ne refusez pas expressément de participer à l'étude, nous considérons que le KCE peut utiliser vos données.

Comment refuser de collaborer à l'étude ?
Vous ne pouvez refuser votre concours qu'en procédant comme suit :

1. Complétez le formulaire de refus ci-annexé : nom + date (voir annexe 1).
2. Signez le formulaire.
3. Envoyez le formulaire à :

L'INAMI – Service des soins de santé - Direction médicale
à l'attention de Marc NÖEL
Avenue de Tervueren, 211
1150 Bruxelles

Avant le 30-04-2009

Vous pouvez utiliser à cet effet l'enveloppe affranchie ci-jointe.
Il ne sera plus tenu compte des formulaires de refus transmis après le 30-04-2009.

Confidentialité

Toutes les données personnelles rassemblées dans le cadre de cette étude seront traitées de manière confidentielle. Les résultats de l'analyse des dossiers seront incorporés dans un rapport du KCE. L'identité des participants ne pourra en aucune manière être retrouvée à partir des éléments du rapport pas plus qu'elle ne sera connue des chercheurs du KCE.
Pour plus d'informations sur la confidentialité de l'étude, prière de consulter l'annexe 2.

...

Personne de contact

Si vous souhaitez un complément d'information ou si vous avez des questions sur l'étude, vous pouvez prendre contact avec Imgard Vinck du KCE, chef de projet.
Téléphone : 02/2873338

Le Fonctionnaire dirigeant
du Service des soins de santé de l'INAMI.

H. DE RIDDER
Directeur général.

Annexes :
1) Formulaire de refus
2) Info sur la confidentialité de l'étude

Annexe 1 : Formulaire de refus sur le FSS

Je confirme avoir lu et compris les informations sur l'étude « Fonds spécial de solidarité » du KCE. Je **ne** suis **pas d'accord** de participer à cette étude.

_____ _____
Date Nom du participant ou de son représentant légal

_____
Signature du participant ou de son représentant légal

Renvoyez ce formulaire **avant le 30-04-2009** à
L'INAMI – Service des soins de santé - Direction médicale
à l'attention de Marc NÖEL
Avenue de Tervueren, 211
1150 Bruxelles

9.3 REPRESENTATIVENESS OF THE SAMPLE

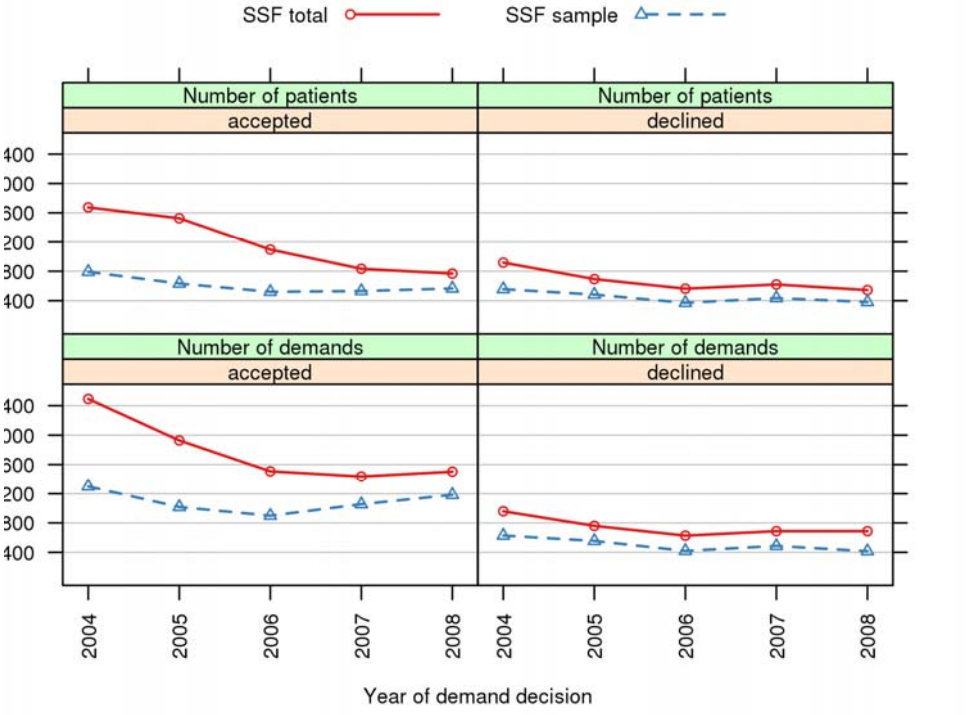
Overall, the representativeness of the sample increases over the years from more than half of the demands introduced in 2004 to over three quarters of the demands introduced in 2008 (see table 13). The evolution of the number of demands between 2004 and 2008 is generally mirrored in the SSF sample, with the exception of 2008 where the decline is less pronounced in the sample.

Table 13: Number of demands in the SSF annual reports and in the SSF sample by year (introduction date).

Year	Number of demands SSF (annual report)	Number of demands SSF (sample)	Percentage sample/total
2004	3529	2040	57.81%
2005	2945	1737	58.98%
2006	2402	1569	65.32%
2007	2524	1831	72.54%
2008	2376	1811	76.22%

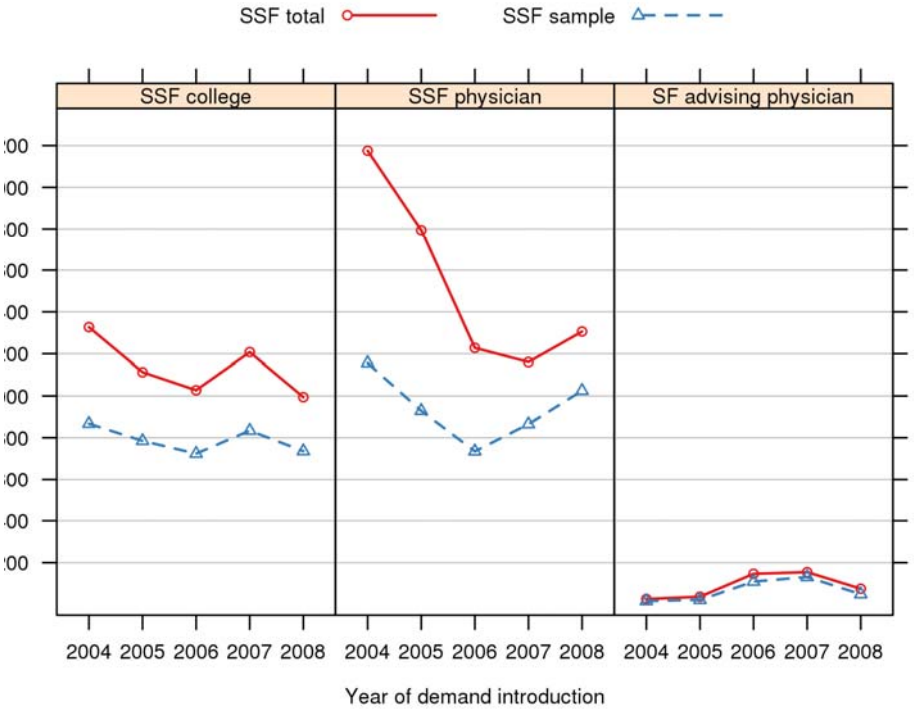
The representativeness of the SSF sample is somewhat less when it concerns the decision taken (see figure 17). For the accepted demands, a decline in the number of demands between 2004 and 2008 was reported by the SSF (see also table 13). In the SSF sample, the pattern is different with a decline between 2004 and 2006, but an increase from 2007 onwards. This suggests that the representativeness of the SSF sample for accepted demands varies between years. For the declined demands, the SSF sample follows the SSF evolution more closely.

Figure 15: Number of demands (bottom panels) and number of patients (top panels) in function of the decision taken by year (decision date).



When viewed by who took the decision to accept or reject the demand, we find a less pronounced increase in representativeness over the years, except for the demands treated by an SSF physician (see figure 16). Overall however, the SSF sample follows the same evolution in number of demands as the SSF in total.

Figure 16: Number of demands in function of the body that took the decision by year (introduction date) (SF: sickness fund).



In terms of the budget spent by the SSF, the sample captures about three quarters of the actual SSF budget (see table 14). Also, the evolution in the SSF sample matches the evolution of the actual SSF budget quite well.

Table 14: Budget spent by the SSF both in total and in the SSF sample by year (decision date).

year	SSF budget spent	SSF sample budget spent	Percentage sample/total
2004	€ 14 998 593	€ 10 206 725	68.05%
2005	€ 7 031 980	€ 4 933 133	70.15%
2006	€ 10 076 402	€ 6 300 248	62.52%
2007	€ 11 661 714	€ 9 429 723	80.86%
2008	€ 8 692 000	€ 6 346 846	73.02%

The time a demand spent at the SSF is reported in the annual reports as the number of working days between reception of the demand and the informing of the insured, with deduction of the time needed to obtain extra information from the insured. In the SSF sample, we tried to reconstruct this information as follows: The difference in working days between the date when the SSF received the demand and the date when the SSF sent a notification about the decision was taken as the total time needed to treat the demand.

This method of calculation results in an overestimation of the total time needed to treat a demand but only when extra information was requested. However, only in 5.57% of the demands between 2004 and 2008, extra information was requested. The large majority of the demands thus have a correct throughput time.

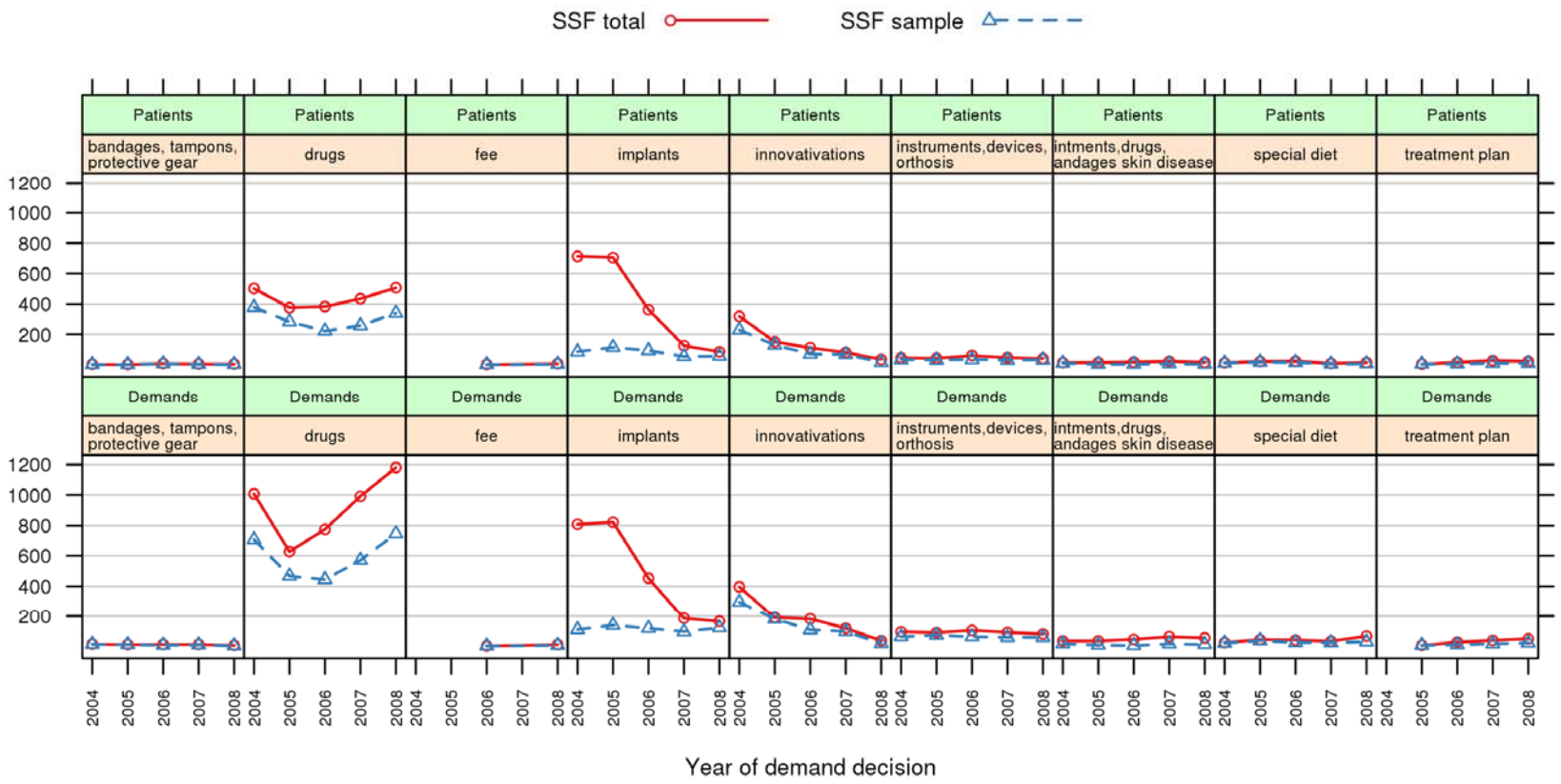
For 2005 to 2007, the average throughput time needed to treat a demand in the SSF sample was much larger than the time reported by the SSF in their annual report (see table 15). For 2008, the average SSF sample throughput time is comparable to the SSF reported throughput time.

Table 15: Throughput time in number of working days needed by the SSF both in total and in the SSF sample by year (decision date).

year	SSF total mean	SSF sample mean	SSF sample median	SSF sample Q1	SSF sample Q3
2004	14.75	10.15	6	2	14
2005	14.3	21.01	10	4	15
2006	14.44	19.37	11	3	15
2007	13	21.05	9	3	14
2008	12.37	12.21	5	2	12

Of the nine types of health care services provided, identified in the SSF annual report, only for the implants does the representativeness of the SSF sample vary widely over years (see figure 17). The other types follow the evolution of the SSF reasonably well.

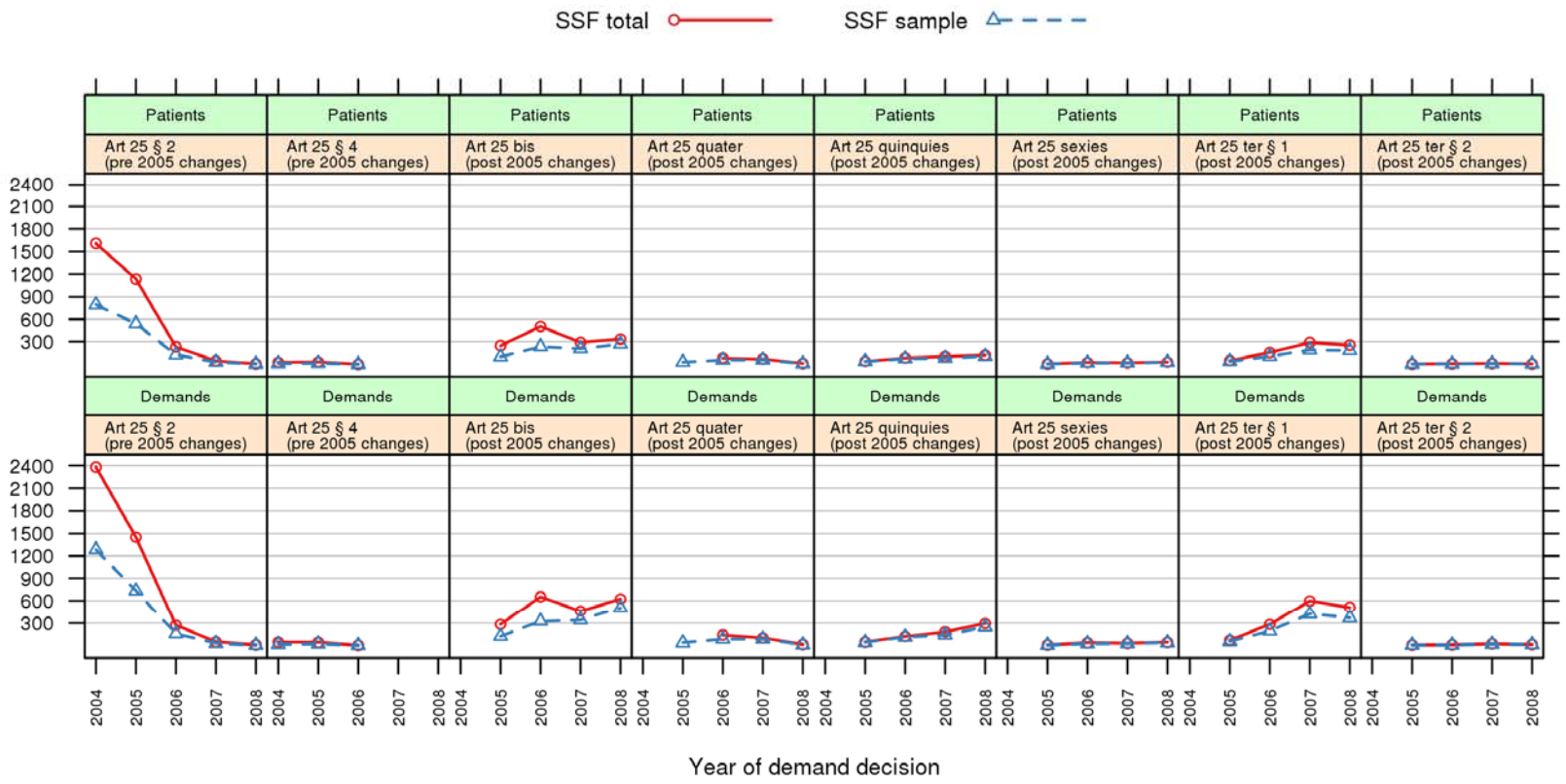
Figure 17: Number of demands (bottom panels) and number of patients (top panels) in function of the type of health care service provided by year (decision date).



Concerning the reason a demand was accepted, the representativeness is fairly good (see figure 19). The apparent decline in representativeness for Article 25 §2 for more recent years is an effect of scale: in 2006 and 2007, the sample contains about half the number of actual number of demands, which is similar to 2004 or 2005. The first two columns in the figure represent the criteria before the change in 2005 and can explain the huge drop in demands for those criteria in more recent years.

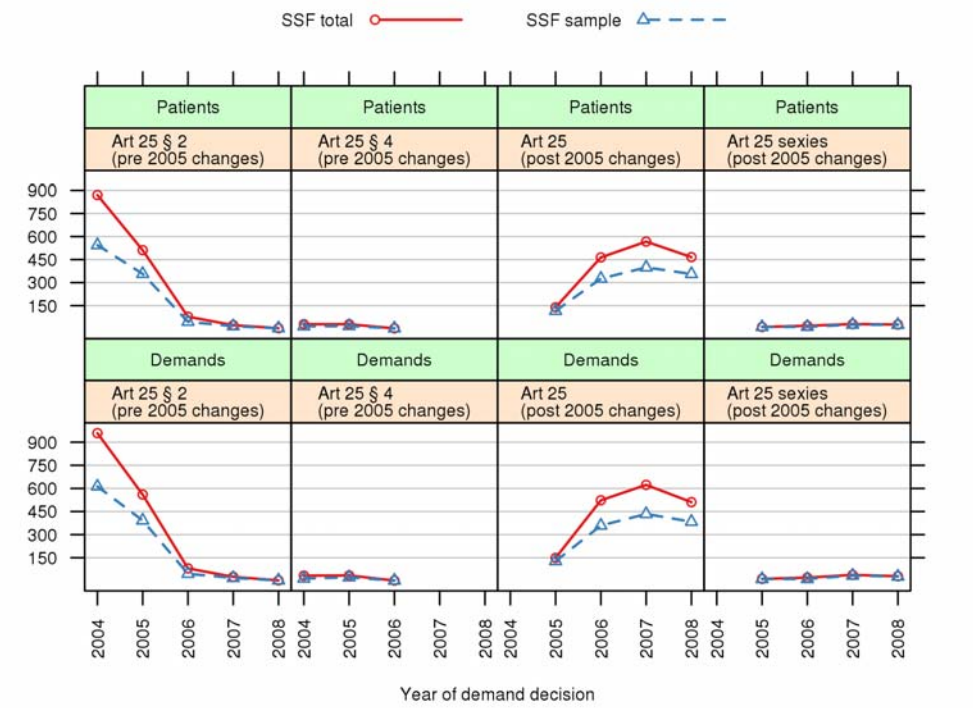
For Article 25quater, no data were available for 2005 in the SSF annual reports, while in the sample a number of demands were identified.

Figure 18: Number of demands (bottom panels) and number of patients (top panels) of accepted demands in function of the reason of the decision taken by year (decision date).



Similarly, for the rejected demands, the representativeness is fairly good (see figure 19). For the rejected demands, all paragraphs of the new Article 25 with the exception of Article 25sexies (from 2005 onwards) are grouped into one Article 25 category in the SSF sample and in the SSF annual reports. Hence, less criteria are available for rejected demands.

Figure 19: Number of demands (bottom panels) and number of patients (top panels) of rejected demands in function of the reason of the decision taken by year (decision date).

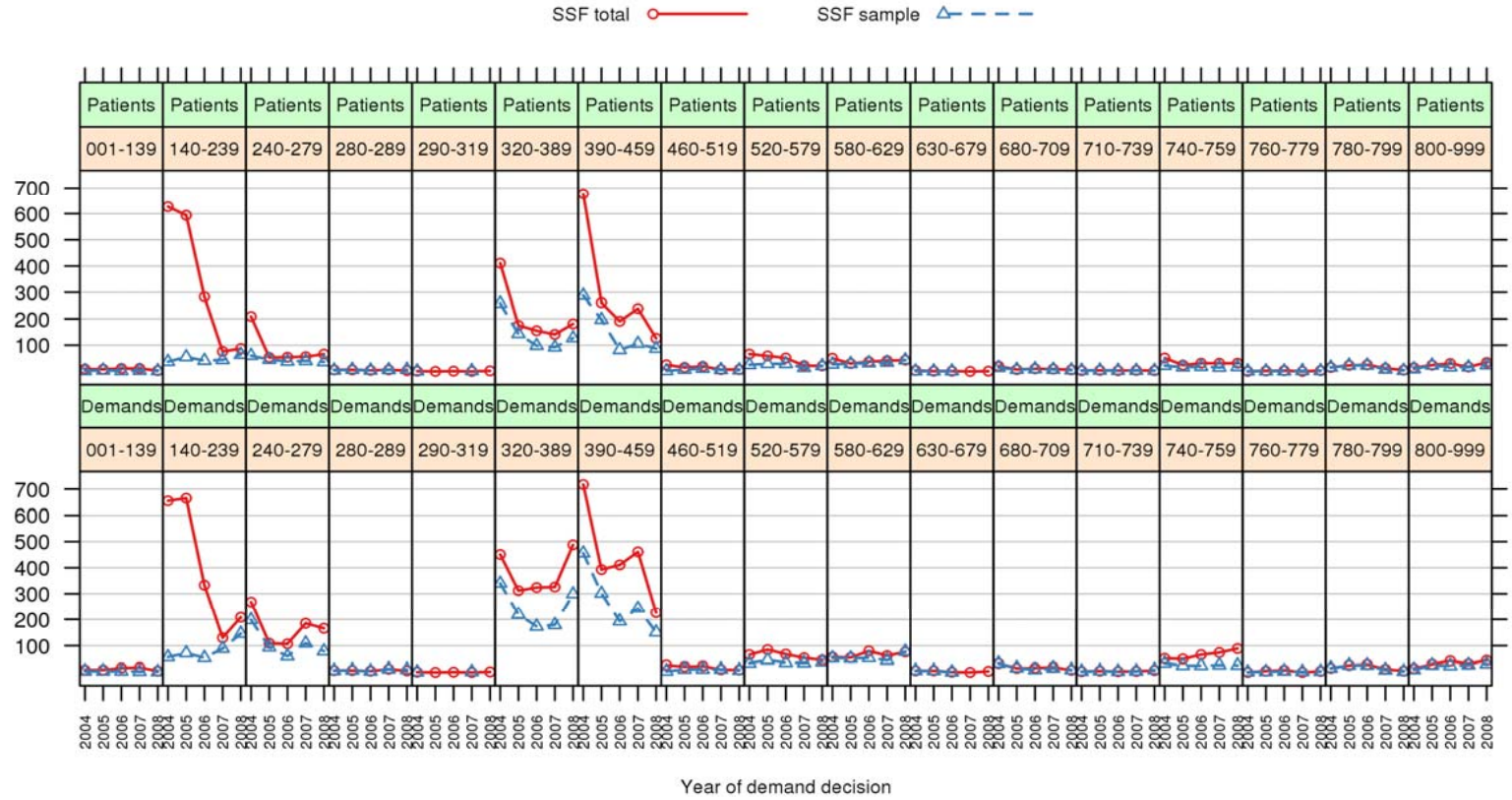


Finally, we ran a representativeness on the number of demands and patients according to the ICD-9-CM diagnosis attributed to the demand. In the SSF annual reports for 2004 up to 2008, only for demands accepted due to Article 25, bis, ter § 1 and ter § 2 was this information available for all years. Figure 22 shows the results by ICD-9-CM three digit group (of which the labels are shown in table 16). For most of the diagnostic groups, the SSF sample follows the SSF actual evolution quite good, with the exception for group 140-239 (neoplasms) in 2004 and 2005.

Table 16: ICD-9-CM three digit group labels

ICD-9-CM	Label
001-139	Infectious and Parasitic Diseases
140-239	Neoplasms
240-279	Endocrine, Nutritional and Metabolic Diseases, and Immunity Disorders
280-289	Diseases of the Blood and Blood-Forming Organs
290-319	Mental Disorders
320-389	Diseases of the Nervous System and Sense Organs
390-459	Diseases of the Circulatory System
460-519	Diseases of the Respiratory System
520-579	Diseases of the Digestive System
580-629	Diseases of the Genitourinary System
630-677	Complications of Pregnancy, Childbirth and the Puerperium
680-709	Diseases of the Skin and Subcutaneous Tissue
710-739	Diseases of the Musculoskeletal System and Connective Tissue
740-759	Congenital Anomalies
760-779	Certain Conditions Originating in the Perinatal Period
780-799	Symptoms, Signs and Ill-defined Conditions
800-999	Injury and Poisoning

Figure 20: Number of demands (bottom panels) and number of patients (top panels) of accepted demands in function of the ICD-9-CM diagnosis by year (decision date).



9.4 SEGMENTATION CRITERIA OF THE SAMPLE SOCIAL SERVICES

To build our theoretical purposal sample, we used the following segmentation (stratification) criteria were:

- The number of applications introduced ('small' - with less than 18 prescriptions versus 'big' with more than 18 applications): the threshold of the number of applications was set according to the percentile 50 of the distribution of the applications >=10
- The setting: university versus non-university hospital: As, in general, reference centers for particular diseases and higher specialization levels are linked to a university hospital, we suppose that there could be a difference in the appeal to and knowledge of the SSF.
  The type of patients, the relationship with patients and complexity of the pathologies could also differ between the settings.
- The native language of the hospital: French or Dutch-speaking

We did not search to reach theoretical saturation but to approach it we have foreseen to interview 1 representative of at least 2 social services per segment, i.e. 16 persons.

Considering our sample grid here under, we tried to complete it gradually based on the list of the potential participants. We therefore recruited social services following the NIHDI data on the prescribing hospital. These were ascendingly sorted based of the number of applications their patients have introduced during the 5 referral years (>=10) and the percentile 50 was calculated. Then they were contacted beginning from the extremes of the list to plan an appointment for the interview until the sampling grid was completed. We noticed that no French- or Flemish-speaking hospital included in the database fitted in the segment university hospital / small applicant. As a consequence, both segments could not be covered, implying that the number of theoretical interviewed persons (16) decreased to 12. Because of the distribution of the number of applications of the available sample (a small number of hospitals presents a large number of applications), the significance of the threshold determined by the percentile 50 will not clearly distinguish the two groups (large and small number of applications). Starting from the extremes we limit the number of social services near to the 50 percentile threshold.

We asked to each service to refer to a person as responsible for the application of interventions asked to the SSF.

Table 17: Segmentation

0			**Theoretical sample**	**Expected sample**	**Realised sample**
Small applicants: 10 to 17 applications between 2004 and 2008	University setting	French-speaking	2		
		Flemish-speaking	2		
	Non university setting	French-speaking	2	2	2
		Flemish-speaking	2	2	2
Big applicants 18 applications or more between 2004 and 2008	University setting	French-speaking	2	2	2
		Flemish-speaking	2	2	2
	Non university setting	French-speaking	2	2	2
		Flemish-speaking	2	2	2
TOTAL			16	12	12

9.5 SEGMENTATION CRITERIA OF THE SAMPLE PRESCRIBING PHYSICIANS

To build our theoretical purposal sample, we used the following segmentation (stratification) criteria:

- The number of applications introduced ('small' prescribers - with less than 18 prescriptions versus 'big' prescribers' - with more than 18 applications): the threshold of the number of applications was set according to the percentile 50 of the distribution of the applications >=10.
- The setting: university hospital, non-university hospital, private practice: justification of the use of the criteria is the same as for the sample of social services. However, an additional setting could be added here, i.e. private practice. Indeed, according to the NIHDI individual data available (for which patients have given their informed consent to be included in the database to analyze 23), about a quarter of the requests for reimbursement have been introduced after a prescription by a private medical physician. The rest of the applications are linked to a 'hospital prescriber'. Moreover, the presence of social services in hospitals and the collaboration with peers could be factors that change perception of the functioning of the SSF.
- The working language of the physicians: French- or Dutch-speaking

We did not search to reach theoretical saturation but to approach it we foresee to interview at least 2 physicians per segment, i.e. 24 persons.

We recruited participating physicians through a list of willing to participate physicians. Indeed, the 62 doctors who have submitted more than 10 applications for compensation by the SSF in 2006 and 2007 (most up-to-date data available at the moment of selection) were asked to participate in the survey by written informed consent (information letter see annex 9.2.). Twenty-two doctors have responded positively. The number of participants needed to perform focus groups was thus too small and confirms the need to perform individual interviews.

The 22 physicians willing to participate were first identified through an internet search. Based on this action, we found that all doctors (22) who had given their consent for participation were associated with a hospital. Consequently, the initial criterion of "private practice" was irrelevant and abandoned and the number of doctors to be interviewed was reduced from 24 (12 segments) to 16 (8 segments). Secondly we noticed that only one doctor, willing to participate, fitted in the segment of university hospital/small applicant/French speaking. In the segment university hospital/small applicant/Flemish speaking two potential doctors were identified but only one interview could take place. In the segment non university/big applicant/French speaking only one interview could take place.

The doctors were contacted by phone to ask them the confirmation of their participation and to schedule an appointment. We used an Excel sheet, where the number of applications was sorted ascendingly and the percentile 50 was calculated. We contacted the doctors as much as possible starting from the extremes.

23 see section on the data analyse

Table 18: Segmentation

			Theoretical sample	Expected sample	Realized sample
Small applicant 10 to 17 applications in 2006 and 2007	University setting	French-speaking	2	1	1
		Flemish-speaking	2	2	1
	Non university setting	French-speaking	2	2	2
		Flemish-speaking	2	2	2
	Private practice	French-speaking	2		
		Flemish-speaking	2		
Big applicant 18 applications or more in 2007 and 2008	University setting	French-speaking	2	2	2
		Flemish-speaking	2	2	2
	Non university setting	French-speaking	2	2	1
		Flemish-speaking	2	2	2
	Private practice	French-speaking	2		
		Flemish-speaking	2		
TOTAL			24	15	13

9.6 INTERVIEW GUIDE MEMBERS OF THE COLLEGE OF MEDICAL DIRECTORS

OPDRACHT EN DOEL BSF:

1. Wat is volgens u het primaire doel van het BSF?
2. Beantwoordt het doel van het BSF aan een reële nood? Hoe zou je die definiëren?
3. Dient het doel van het BSF aangepast – uitgebreid – beperkt te worden? (zo ja, licht toe)
4. Heeft u zicht op bepaalde groepen die niet of onvoldoende bereikt worden? In welke mate zijn er regionale verschillen? Verschillen tussen of binnen bepaalde pathologiegroepen?
5. Waarop ligt volgens u de klemtoon vandaag? Op de solidariteit (in geval zware kosten behandeling)?Op de vergoedbaarheid van kosten (die niet vergoed worden door de verplichte ziekteverzekering) ivm zeldzame indicaties-aandoeningen-innovatieve medische technieken –chronisch zieke kinderen? Andere?
6. Bent u op de hoogte van buitenlandse mechanismen die een gelijkaardige rol als het BSF vervullen, Zo ja, welke?

INTERNE FASE VAN BEHANDELING DOSSIERS BSF BINNEN HET ZIEKENFONDS:

Hoe verloopt de interne procedure van behandeling van dossiers voor het BSF?

Wat zijn de verschillende stappen die doorlopen worden?– Beschrijf deze schematisch en geef het niveau aan waarop deze zich voordoen. (vb. lokaal niveau, niveau ziekenfonds, niveau landsbond – nationaal verbond)

Niveau ziekenfonds:

1. Wie initieert een dossier?
2. Wie behandelt een aanvraag voor tegemoetkoming?
   a. Zijn dit meerdere personen (bv. administratieve dienst, sociale dienst, adviserend geneesheer)?
   b. Is er één adviserend geneesheer op het ziekenfonds niveau specifiek belast met de aanvragen BSF?
   c. of behandelen de verschillende adviserende geneesheren ieder hun aanvragen?
   d. In voorkomend geval, hoe worden de aanvragen aan de verschillende adviserende geneesheren toebedeeld?
3. Worden er op het niveau ziekenfonds criteria van selectie gehanteerd? Zo ja, welke?
4. Wat wordt er eventueel ondernomen om het dossier te stofferen, aan te vullen, te ondersteunen?
5. Hoe wordt de patiënt bij de opstelling van zijn dossier betrokken?
6. Hoe wordt de volledigheid van de noodzakelijke gegevens in het aanvraagdossier gewaarborgd (vb. anamnese…)?
   a. Zijn er hiervoor interne richtlijnen – checklists die gehanteerd worden?
   b. Zo ja, welke?
7. Op welke wijze wordt binnen het ziekenfonds nagegaan of er in hoofde van de patiënt geen andere rechten op terugbetaling bestaan? (Vergoedbaarheid in kader verplichte ziekteverzekering, vergoedbaarheid in kader van arbeidsongeval of beroepsziekte, vergoedbaarheid in kader private verzekering patiënt (eventueel hospitalisatieverzekering….)
8. Wie neemt de beslissing over al dan niet overmaken van de aanvraag aan de landsbond – nationaal verbond?
   a. Worden er bij deze beslissing criteria gehanteerd?
   b. Zijn er uitgeschreven richtlijnen? Zo ja, welke?
9. Welke informatie wordt er tijdens de loop van de procedure aan de patiënt doorgegeven?

Niveau landsbond- nationaal verbond:

1. Wie onderzoekt op het niveau Landsbond – nationaal verbond de door de adviserende geneesheren (ziekenfondsen) overgemaakte dossiers?
   a. Zijn dit meerdere personen / diensten (bv. administratieve dienst, sociale dienst, Geneesheren)?
   b. Is er één geneesheer op het niveau landsbond – nationaal verbond specifiek belast met de aanvragen BSF?
   c. Of behandelen de verschillende geneesheren elk hun aanvragen?

d. In voorkomend geval, hoe worden de aanvragen aan de verschillende geneesheren toebedeeld?

2. Worden er op het niveau Landsbond – nationaal verbond criteria van selectie gehanteerd; zo ja welke?
3. Hoe wordt de volledigheid van de noodzakelijke gegevens in het aanvraagdossier gewaarborgd? (vb anamnese…)
   a. Zijn er hiervoor interne richtlijnen – checklists die gehanteerd worden?
   b. Zo ja, welke?
4. Op welke wijze wordt binnen de landsbond – nationaal verbond nagegaan of er in hoofde van de patiënt geen andere rechten op terugbetaling bestaan? (Vergoedbaarheid in kader verplichte ziekteverzekering, vergoedbaarheid in kader van arbeidsongeval of beroepsziekte, vergoedbaarheid in kader private verzekering patiënt (eventueel hospitalisatieverzekering….)
5. Wie neemt de beslissing over het al dan niet indienen van het dossier bij het BSF?
6. Worden er bij de beslissing criteria gehanteerd?
   a. Zijn er uitgeschreven richtlijnen?
   b. Zo ja, welke?
7. Worden aanvraagdossiers op voorhand - voor indiening - met de administratie van het BSF besproken?
   a. Worden aanvraagdossiers na indiening met de administratie BSF besproken?
   b. Is er feedback naar de initiële dossierbehandelaar binnen het ziekenfonds (adviserend geneesheer) over de beslissing van het College of het BSF?
   c. Welke feedback wordt er aan de patiënt gegeven?

Kwantitatieve gegevens:

1. Hoeveel aanvragen voor tegemoetkoming BSF ontvangt de mutualiteit (nationaal niveau landsbond – nationaal verbond per jaar? (initiële aanvragen)
2. Zijn deze gegevens qua aantallen per regio (ziekenfondsen) beschikbaar?
3. Hoeveel van de initiële aanvragen worden uiteindelijk bij het BSF ingediend?
4. Wat is de gemiddelde doorlooptijd van een aanvraagdossier binnen de mutualiteit (vanaf ontvangst niveau ziekenfonds tot indiening bij het BSF)?

Werking college van Geneesheren-Directeurs:

Hoeveel verschillende personen nemen vanuit uw landsbond– nationaal verbond deel aan de vergaderingen van het college?

Indien meerdere:

1. Wat zijn de criteria tot deelname (de concreet geagendeerde dossiers – tijdsaspect…)?
2. In welke verhouding nemen de verschillende personen deel aan de vergaderingen?
   a. Wordt de vergadering van het college voorafgaandelijk intern voorbereid?
   b. Zo ja, hoe?
3. Is er betrokkenheid van de adviserend geneesheer (niveau ziekenfondsen)?
4. Worden alle dossiers onderzocht en voorbereid, of enkel deze van het eigen ziekenfonds?
   a. Worden er over concrete dossiers bijkomende inlichtingen bij het BSF opgevraagd?

Zo ja, welk type van informatie?

5. Hoe frequent is dit (zelden-regelmatig-steeds)?
   a. Zijn er uitgeschreven criteria voor het nemen van beslissingen over aanvragen door het college? (buiten de wettekst - vb. beslissingsboom)

   Zo ja, welke;

6. Zo neen, acht u dergelijke uitwerking noodzakelijk – wenselijk?
   a. Welke elementen acht U noodzakelijk en dienstig om de beslissingen te nemen?
   b. In welke mate zijn de in de wet gehanteerde voorwaarden en criteria voldoende duidelijk omschreven? (licht toe aub - waar eventueel wel –waar niet?
   c. In welke mate zijn de criteria aangepast aan de doelstellingen van het BSF (licht toe aub)?
   d. Wordt er voldoende tijd gegeven om de dossiers voor te bereiden?
   e. Wordt er voldoende tijd uitgetrokken om de dossiers te bespreken en een adequate beslissing te nemen?
   f. Worden er naast dossiers ook principiële beslissingen genomen?
   g. Zo ja, van welk type?
   7. Zo neen, bestaat er behoefte om gedragslijnen inzake beslissingen te bespreken en vast te leggen?

Procedure behandeling dossiers binnen de administratie BSF:

Hoe verloopt volgens U de interne behandeling van de dossiers binnen de diensten van het BSF?

Hoe beoordeelt U deze interne procedure?

1. In welke mate is de interne behandeling transparant?
2. Zijn er volgens U tekortkomingen?
3. Zo ja, welke?

Op welke basis wordt beslist een dossier op het college te behandelen, dan wel te delegeren aan een lid van het college of aan de VI's? Wat zijn de toegepaste criteria voor deze keuze?:

1. Dossiers die op het college dienen voorgebracht te worden
2. Dossiers die aan een lid van het college worden gedelegeerd
3. Dossiers die aan de VI's worden gedelegeerd

Wat is uw oordeel over de actuele delegaties naar zowel het lid van het college als naar de VI's? (licht toe aub)

In welke mate zijn de delegaties eventueel te ruim of te eng?

Aan een lid van het college gedelegeerde dossiers BSF:

Hoe gebeurt de rapportering aan het college van de in het kader van de delegatie genomen beslissingen?

Hoe wordt de controle op de beslissingen die in het kader van de delegatie worden genomen georganiseerd?

In welke mate is de controle sluitend, adequaat en noodzakelijk?

Aan de VI's gedelegeerde dossiers BSF:

In welke mate is er een georganiseerde controle op de beslissingen die in het kader van de delegatie door de verschillende VI's worden genomen? Hoe wordt deze controle georganiseerd?

In welke mate is deze controle sluitend, adequaat en noodzakelijk?

Wie neemt in uw ziekenfonds de beslissingen over de dossiers die in het kader van de delegatie worden genomen?

1. Op welk niveau worden deze genomen (Verbond – Landsbond)?
2. Is er binnen uw ziekenfonds post controle op deze beslissingen? Zo ja, hoe is deze georganiseerd?

Hoe worden deze dossiers intern behandeld (stappen – betrokken diensten)?

Hoeveel dossiers werden in het kader van de delegatie over 2007 behandeld? Hoeveel goedgekeurd – afgewezen?

Wat is de gemiddelde doorlooptijd van de dossiers (aanvraag tot beslissing)?

Beschikt u over een systematisch overzicht (aard, aantallen, uitgaven..)van de in het kader van de delegatie behandelde dossiers?

Hoe organiseert uw ziekenfonds de feedback aan de diensten van het BSF inzake de in het kader van deze delegatie genomen beslissingen? (aard van beslissingen en financiële impact)

Wat is de 'reële' gemiddelde termijn van indiening van de uitgavenstaat van de in het kader van de delegatie goedgekeurde dossiers? Hoeveel staten werden over 2007 ingediend?

Budget BSF:

Hoe wordt het budget voor het BSF jaarlijks vastgesteld?

Welke criteria worden gebruikt voor berekening van dit budget? Wat denkt U van deze criteria en in welke mate beantwoorden ze al dan niet aan de noodwendigheden?

Welke gegevens heeft U op het niveau van uw ziekenfonds inzake reële behoeften aan tussenkomsten door het BSF?

In welke mate heeft de omvang van het jaarlijks budget van het BSF de facto een impact op de mate van goedkeuring van aanvraagdossiers?

Voorwaarden en criteria inzake tegemoetkoming via het BSF:

Welke definitie hanteert U voor

- "zeldzame aandoening"?
- "zeldzame indicatie"?
- "innovatieve techniek"? "zeldzame medische hulpmiddelen en verstrekkingen"
- "aangetoonde meerwaarde na kosten-baten afweging verstrekkingen die innovatieve technieken zijn"
- "Bewezen wetenschappelijke waarde en effectiviteit"
- "Bedreiging van vitale levensfuncties"
- "het experimenteel stadium" voorbij?
- "Duur"
  - De verstrekking is "duur"
  - De verzorging is in haar totaliteit "duur"
  - De medische hulpmiddelen en verstrekkingen zijn "duur"

- Is het inkomen van de betrokken patiënt een element in de beoordeling van het begrip "duur"? Zo ja, wie beoordeelt dit?
- "Behartenswaardige gevallen" (medische kosten van in het buitenland verleende zorg)

In welke mate zijn de voorwaarden en criteria zoals opgenomen in de wet:

- Voldoende duidelijk? Hoe zou desgevallend meer duidelijkheid kunnen tot stand gebracht worden?
- Aangepast aan de reële behoeften; Waar is desgevallend bijsturing wenselijk – noodzakelijk?
- In welke mate zijnde in de wet vermelde criteria en voorwaarden volledig/onvolledig?

Beschikt uw mutualiteit over eigen studies, nota's, informatie, publicaties (zowel interne als externe) inzake de tegemoetkomingen door het BSF? Zo ja, graag bezorgen.

Heeft uw ziekenfonds met betrekking tot het BSF contacten met:

- Artsenverenigingen?
- Patiëntenorganisaties?
- Ziekenhuizen? Gespecialiseerde centra?
- Andere belanghebbenden?

Zo ja, met welke?

Welke lijken U belangrijk om in het kader van deze studie te contacteren?

9.7 INTERVIEW GUIDE SOCIAL SERVICES

9.7.1 Dutch version

Duurtijd (+ formulering vragens*)	**Vragen**	**Hulp bij vragen**
	Introductie tot onderhoud	
.	Welkom + dank voor aanwezigheid Voorstelling + functies van moderator/verslaggever Doel van de studie Toelichting wijze van selectie deelnemers Verwachtingen ten opzichte van de deelnemer Er zijn geen juiste of foute antwoorden : de visie van elke deelnemer is belangrijk Toelating vragen om gesprek op te nemen (doel : minder nota's moeten nemen en antwoorden niet vervormd registreren) Confidentialiteit (o.m. nota's en opname die achteraf vernietigd wordt) resultaten interviews komen in rapport dat beschikbaar zal zijn Januari 2010	**Objectieven van de studie** perceptie nagaan van de nood om over een financieel vangnet voor belangrijke medische kosten die niet door de verplichte ziekteverzekering gedekt worden, te beschikken. Andere strategieën en kanalen dan het BSF, gebruikt om deze soort uitgaven te dekken Kennis over het BSF en de functionering ervan pertinentie en efficiëntie van het BSF (inbegrepen tekorten op vlak van de tussenkomsten) werking van het BSF en implicaties voor de respondent (procedure, criteria voor tussenkomst, beschikbare informatie, mogelijkheid tot aantekenen beroep, communicatieproces, positieve en negatieve elementen) wijze waarop de respondenten gebruik maken van het BSF (eigen criteria, administratieve belasting,e.d.)

	Inleidende vragen / informatie over respondent	
	1. Kan U kort de werking van uw sociale dienst alsook uw rol hierin toelichten ?	- Hoeveel FTE? - Hoeveel dossiers worden per jaar behandeld?
	<u>Verhouding tot het BSF - context</u> 2. De laatste jaren werden er verschillende aanvragen tot tussenkomst van het BSF ingediend die afkomstig zijn van Uw ziekenhuis. a. In globo, hoeveel aanvragen dient U per jaar in? b. Hoeveel aanvragen die NIET leiden tot aan aanvraag tot tussenkomst BSF worden er door de sociale dienst behandeld c. Voor beide gevallen, op hoeveel patiënten hebben deze betrekking?	

	d. Van hoeveel (ongeveer) artsen komen deze dossiers voort? e. Welk zijn de indicaties, pathologieën, aandoeningen of types van kosten waarvoor U aanvragen bij het BSF indient?	
	Noodzaak aan vangnet /relevantie van BSF/ effictiviteit van BSF	
	1. Waarom werd volgens U bovenop de verplichte verzekering het BSF als vangnet opgericht? De belangrijkste doelstelling van het BSF bestaat erin om financieel tussen te komen teneinde te voorkomen dat patiënten, in geval van een zeer ernstige medische toestand, aan zorgen zouden moeten verzaken, wanneer één of meerdere essentiële medische prestaties niet vergoed wordt(en) en deze prestatie(s) duur zijn (is). Deze (vraag tot) tussenkomst moet aan een aantal criteria beatwoorden. 2. In welke mate bereikt/haalt het BSF volgens U deze doelstelling? 3. Aan wat wijdt U de 'niet gehaalde' aspecten (van de doelstelling)? 4. Welke andere mogelijkheden ziet U om een antwoord te geven op dit type van behoefte?	- Wat denkt U van dergelijk specifiek afzonderlijk systeem? - Welke aspecten (van de doelstelling) worden goed bereikt? - Welke aspecten (van de doelstelling) worden niet gehaald - (ander) Afzonderlijk systeem? - Binnen het systeem van de verplichte verzekering?
	andere strategiën	
	5. Doet U in de praktijk beroep op andere (alternatieven) middelen (buiten het BSF) om de kosten die niet door de verplichte verzekering gedekt worden te vergoeden? 6. In welke mate zijn deze alternatieven te veralgemenen en integreerbaar in een meer algemeen systeem?	
	Pertinentie van het BSF	
	De interventiedomeinen betreffen: → *Tekst brochure tonen* □□de zeldzame indicaties □□de zeldzame ziekten	

	□□de zeldzame ziekten die continue en complexe zorgen vereisen □□de verstrekkingen die innovatieve medische technieken zijn - met uitsluiting van geneesmiddelen □□de chronisch zieke kinderen □□ de in het buitenland verleende geneeskundige verstrekkingen 7. Welke andere elementen zouden eventueel door het BSF gedekt moeten worden? 8. Welke elementen zouden volgens U door dergelijk systeem niet gedekt moeten worden?	
	Kennis van het bestaan van het BSF	
	9. Hoe hebt U kennis gekregen van het bestaan en de functionering van het BSF?	
	Ervaring van de verstrekker met het BSF / kennis over de werking van het BSF	
	10. Kan U beschrijven hoe het indienen van een aanvraag volgens U verloopt ? vanaf de identificatie van een eventuele behoefte tot de uiteindelijke financiële tussenkomst door het BSF?	
	11. a. Volgens welke criteria kan een dossier volgens U in aanmerking komen voor een tussenkomst vanuit het BSF? *! De respondent uitnodigen om de aangehaalde criteria te detailleren* *bv : « duur » → « vanaf wanneer is iets duur ? » ; « voor bepaalde pathologiën » → «welke pathologie » …*	- Wanneer en hoe beslist U om een aanvraag tot tussenkomst van het BSF voor te stellen?
	b. Van wie komt het initiële idee/voorstel om een aanvraag tot tussenkomst van het BSF in te dienen?	Patiënt/ arts /sociale dienst / andere
	c.In welke omstandigheden, indien dit zich voordoet, adviseert U een patiënt (of een arts) om GEEN aanvraag tot tussenkomst van het BSF in te dienen?	
	d. Wie neemt de finale beslissing om al dan niet een aanvraag tot tussenkomst in te dienen?	Patiënt/ arts /sociale dienst / andere
	e. Wat gebeurt er concreet wanneer beslist werd om een aanvraag in te dienen?	Wat is het traject van de aanvraag? - Wie stelt ze op?

	f.In welke stappen komt Uw dienst tussen en op welk vlak?	- Naar waar gaan de aanvraag? - Wie verstuurt ze? - Aan wie wordt ze verstuurd? - Welke zijn de volgende stappen?
	g.In welke mate wordt U, na indiening van een aanvraag, op de hoogte gehouden van het verdere verloop van het (aanvraag) dossier?	- Opstelling aanvraag? - verbetering / aanpassing aanvraag? - Bijkomende inlichtingen aan de sociale dienst van het ziekenfonds, de diensten van het BSF? - Wie informeert U? - Welke soort informatie verkrijgt U? - Motivering van de beslissing? - Wie wordt 'ook' geïnformeerd? - Door wie?
	12. Bij welke stappen in de procedure zou U, in voorkomend geval, meer betrokken willen zijn in het proces? Waarom? Hoe?	- Informatie over de motivering van de beslissing?
	13. Van welke elementen in het proces zou U, in voorkomend geval, en indien mogelijk ontlast willen worden? Waarom? Hoe?	- wie zou dit moeten overnemen?
	14. Wat is in het algemeen de 'uitkomst' van uw dossiers BSF?	
	15. Welke informatie met betrekking op de slaagkans van de aanvraag geeft U aan de patiënt? (voorafgaandelijk aan de beslissing BSF)?	
	16. Wat kan U volgens U doen indien een aanvraag tot tussenkomst geweigerd wordt?	Internet/ RIZIV/ sociale dienst/ andere
	17. Hoe wordt U geïnformeerd over evoluties (wijzigingen) in de regelgeving qua tussenkomst van het BSF?	Duidelijkheid/ volledigheid/ andere
	18. Hoe beoordeelt U de kwaliteit van de beschikbare informatie rond het BSF?	Op gebied van: - duidelijkheid

	19. Tenslotte, hoe beoordeelt U het geheel van de procedure?	-complexiteit - administratieve belasting - bestede tijd - tijdsverloop - doelmatigheid - snelheid - communicatie - andere

	Functionering van het BSF	
	Modaliteiten van functioneren van het BSF **<u>Beslissingscriteria voor tussenkomst vanuit het BSF</u>** Zie de lijst van criteria voor tussenkomst vanuit het BSF zoals door het BSF meegedeeld. → *Grid tonen* 20. Wat is uw appreciatie voor elk van deze criteria in termen van <u>relevantie</u> (beantwoordend aan een reële nood), <u>duidelijkheid</u> en <u>formulering</u>? **<u>Algemene evaluatie van het BSF</u>** 21. Welke positieve punten identificeert U in het bestaan en de werking van het BSF? 22. Welke negatieve punten identificeert U? 23. Welke wijzigingen of verbeteringen zou U voorstellen?	▪ Uniformiteit: contradicties in de beslissingen? - In het systeem zoals het thans bestaat - Wijzigingen op het vlak van het systeem - Hoe kan het gebruik van het BSF verhoogd worden?

9.7.2 French version

Durée (+ formulation des questions*)	**Questions**	**Aide aux questions**
	Introduction à l'entretien	
.	- bienvenue + remerciement pour la présence - présentation + fonction des modérateur / rapporteur - but de l'étude - Explication de la sélection du participant - attentes vis-à-vis du participant * il n'y a pas de bonnes ou mauvaises réponses: la vision de chacun est importante - demander l'autorisation pour enregistrer (but : prendre moins de note et ne pas déformer les propos) - confidentialité (entre autres matériel d'enregistrement, notes) - résultats des entretiens : rapport disponible en janvier 2010	**Objectifs de l'étude** - perception du besoin de disposer d'un filet de sécurité financier pour les dépenses médicales onéreuses qui ne sont pas couvertes par l'assurance obligatoire - Autres stratégies et autres canaux utilisés que le FSS pour couvrir ce type de dépenses - Mode de connaissance du FSS et de son fonctionnement - pertinence et efficacité du FSS (dont les manques dans les interventions) - fonctionnement du FSS et implications pour le répondant (procédure, critère d'intervention, information disponible, possibilité de faire appel, processus de communication, éléments positifs et négatifs) - manière dont les répondants utilisent le FSS (critères propres, charge administrative, etc.)

	Questions d'ouverture / informations sur le répondant	
	1. Pouvez-vous m'expliquer en quelques mots comment fonctionne votre service ainsi que votre rôle au sein de ce dernier?	- Combien d'ETP? - Combien de dossiers traités par an ?
	<u>Rapport au FSS – contexte</u> 2. Les années passées, plusieurs demandes d'interventions au FSS provenant de votre hôpital ont .été introduites	

	2.1. Globalement, d'après vous, combien de demandes passent par votre service par an? 2.2. Combien de demandes sont traitées qui ne donnent finalement pas lieu à une intervention? 2.3. Dans les deux cas, ces demandes concernent combien de patients? 2.4. Ces demandes proviennent de combien de médecins environ? 2.5. Quelles sont les indications, pathologies ou affections ou type de coûts pour lesquelles ces demandes au FSS sont introduites?	
	Besoin d'un filet de securité /pertinence du FSS/ efficacité du FSS	
	3. D'après vous, pourquoi un système de filet de sécurité supplémentaire au système d'assurance obligatoire tel que le FSS a-t-il été crée ? Le FSS a pour objectif principal d'intervenir financièrement pour éviter qu'un patient, dans une situation médicale très grave, doive renoncer aux soins si une ou des prestation(s) médicale(s) essentielle(s) n'est ou ne sont pas remboursée(s) et est ou sont particulièrement chère(s). Cette intervention doit répondre à certains critères. 4. D'après vous, dans quelle mesure le FSS remplit-il cet objectif? 5. A quoi attribuez-vous ces « éléments manqués »? 6. En théorie, quelles autres possibilités voyez-vous pour répondre à ce type de besoin?	Que pensez-vous d'un tel système spécifique? - Quels sont les aspects qu'il remplit bien ? - Quels sont les aspects non atteints ? - Système à part? - Dans le système d'assurance obligatoire?
	autres stratégies	
	7. En pratique utilisez-vous d'autres moyens pour couvrir des coûts qui ne sont pas couverts par l'assurance obligatoire? 8. Dans quelle mesure ces stratégies sont –elles d'après vous généralisables, transposable dans un système plus global?	
	Pertinence du FSS	

	Les domaines d'interventions concernent : → *MONTRER LE TEXTE DE LA BROCHURE* □□les indications rares □□les maladies rares □□les maladies rares qui nécessitent des soins continus et complexes □□les dispositifs médicaux et/ou prestations qui sont des techniques médicales innovantes - à l'exclusion des médicaments □□les enfants malades chroniques □□ les soins délivrés à l'étranger 9. Quels autres éléments devrait-il éventuellement couvrir? 10. Quels éléments devraient éventuellement selon vous ne pas être couverts par un tel système?	
	Connaissance de l'existence du FSS	
	11. Comment avez-vous eu connaissance du FSS et de son fonctionnement ?	
	Expérience du prestataire du FSS / connaissance du fonctionnement du FSS	
	12. Racontez-moi comment se déroule de votre point de vue l'introduction d'une demande d'intervention depuis l'identification d'un éventuel besoin jusqu'à l'intervention financière? 13. a. D'après vous, selon quels critères un dossier peut-il être éligible pour une intervention du FSS ? *! Inviter le répondant à détailler le contenu des critères qu'il cite par ex : « cher » → « à partir de quand est-ce cher ? » ; « pour certaines pathologies » → « quelle pathologie » …* b. De qui provient l'idée initiale de demander une intervention du FSS ? c. Dans quelles circonstances, s'il en est, vous arrive-t-il de conseiller à un patient ou un médecin de ne pas introduire une demande d'intervention par le FSS ? d. Qui prend la décision finale d'introduire une demande d'intervention ? e. Concrètement, une fois la décision prise de demander une intervention, que se	- Quand et comment décidez-vous de proposer de demander une intervention au FSS? Patient/ médecin / service social / autre Patient/ médecin/service social/ autre

	passe-t-il?	Quel est le trajet de la demande? - Qui la rédige? - Où va-t-elle? - Qui l'envoie? - A qui est-elle envoyée? - Quelles sont les étapes ensuite?
	f. À quelle(s) étape(s) intervient votre service et en quoi?	- Rédaction? - Correction? - Information complémentaire auprès du médecin? de la mutuelle? du FSS?
	g. Après introduction de la demande, dans quelle mesure êtes-vous informé de la suite du dossier?	- Qui informe? - Quel type d'information? - Motivation de la décision? - Qui d'autre est informé? - Par qui?
	14. Le cas échéant et si cela était possible, pour quelles étapes souhaiteriez-vous être plus impliqué dans le processus ? Pourquoi? Comment?	
	15. Le cas échéant et si cela était possible de quels éléments souhaiteriez-vous être déchargé dans le processus ? Pourquoi? Comment?	- Information sur la motivation de la décision? - Qui d'autre devrait s'en charger?
	16. Quelle est en général l'issue de vos dossiers?	
	17. Quelle information relative au succès éventuel de la demande donnez-vous au patient préalablement à la décision?	
	18. Si une demande d'intervention venait à être refusée, d'après vous, que pouvez-vous faire?	

	19. Comment êtes-vous informé des évolutions des règles de remboursement du FSS?	Internet/ INAMI/ service social/ autre
	20. Comment jugez-vous la qualité de l'information disponible à propos du FSS?	Clarté/ complétude/ autre
	21. Enfin comment jugez-vous l'ensemble de la procédure?	En termes de: - clarté - complexité - lourdeur administrative - temps à consacrer - délais - efficacité - rapidité - communication - autre
	Fonctionnement du FSS	
	Parlons maintenant des modalités de fonctionnement du FSS **<u>Critères de décisions pour intervention du FSS</u>** Voici la liste des critères d'éligibilité annoncés par le FSS. → *MONTRER LA GRILLE* 22. Quelle est votre appréciation pour chacun d'entre eux en termes de <u>relevance</u> (répond à un besoin réel), de <u>clarté</u> et de <u>formulation</u>?	
	<u>Evaluation générale du FSS</u> 23. Finalement, quels points positifs identifiez-vous dans l'existence et le fonctionnement du FSS?	Uniformité: contradictions dans les décisions?
	24. Quels points négatifs identifiez-vous?	- Dans le système tel qu'il existe - Changement de système
	25. Quelles modifications ou améliorations suggèreriez vous?	- comment augmenter l'utilisation?

9.8 INTERVIEW GUIDE PATIENT ORGANISATIONS

Duurtijd (+ formulering vragens*)	**Vragen**	**Hulp bij vragen**
	Introductie tot onderhoud	
.	- Welkom + dank voor aanwezigheid - Voorstelling + functies van moderator/verslaggever - Doel van de studie - Toelichting wijze van selectie deelnemers - Verwachtingen ten opzichte van de deelnemer * Er zijn geen juiste of foute antwoorden: de visie van elke deelnemer is belangrijk - Toelating vragen om gesprek op te nemen (doel: minder nota's moeten nemen en antwoorden niet vervormd registreren) - Confidentialiteit (o.m. nota's en opname die achteraf vernietigd wordt) - Resultaten interviews komen in rapport dat beschikbaar zal zijn Januari 2010	**Objectieven van de studie** - Perceptie nagaan van de nood om over een financieel vangnet voor belangrijke medische kosten die niet door de verplichte ziekteverzekering gedekt worden, te beschikken. - Andere strategieën en kanalen dan het BSF, gebruikt om deze soort uitgaven te dekken - Kennis over het BSF en de functionering ervan - Pertinentie en efficiëntie van het BSF (inbegrepen tekorten op vlak van de tussenkomsten) - Werking van het BSF en implicaties voor de respondent (procedure, criteria voor tussenkomst, beschikbare informatie, mogelijkheid tot aantekenen beroep, communicatieproces, positieve en negatieve elementen) - Wijze waarop de respondenten gebruik maken van het BSF (eigen criteria, administratieve belasting, e.d.)
	INLEIDENDE VRAGEN / INFORMATIE OVER RESPONDENT	
	1. Kan U kort de werking van uw organisatie alsook uw rol hierin toelichten?	- Structuur? - Vertegenwoordigde instanties en/of groepen? - Tot welke soort patiënten richt u zich? - Hoeveel leden+/-? - Sinds wanneer bestaat uw vereniging? - Over welke financieringsbronnen beschikt U? - Welke acties hebt U reeds ondernomen (welke resultaten)?
	Wat weet U van het BSF?	

	Wat is volgens U de rol van het BSF?	Waarom is het BSF opgericht?
	Verhouding tot het BSF - context 2. Op welke wijze wordt er door de patiënten die U vertegenwoordigd gebruik gemaakt van het BSF teneinde tussenkomst in de financiële kosten van de ziekten te bekomen? 2.1. In globo, over welk aantal aanvragen gaat het per jaar? 2.2. Op hoeveel patiënten hebben deze aanvragen betrekking? 2.3. Over welke soorten indicaties of ziekten gaat het? 2.4. Over welke soort kosten gaat het? 2.5. Van welke specialisten (voorschrijvende artsen) komen deze dossiers voort? (discipline)	

	NOODZAAK AAN VANGNET /RELEVANTIE VAN BSF/ EFFECTIVITEIT VAN BSF	
	3. Waarom werd volgens U bovenop de verplichte verzekering het BSF als vangnet opgericht? De belangrijkste doelstelling van het BSF bestaat erin om financieel tussen te komen teneinde te voorkomen dat patiënten, in geval van een zeer ernstige medische toestand, aan zorgen zouden moeten verzaken, wanneer één of meerdere essentiële medische prestaties niet vergoed wordt(en) en deze prestatie(s) duur zijn (is). Deze (vraag tot) tussenkomst moet aan een aantal criteria beantwoorden.	Wat denkt U van dergelijk specifiek afzonderlijk systeem?
	4. In welke mate bereikt/haalt het BSF volgens U deze doelstellingen?	- Welke aspecten (van de doelstelling) worden goed bereikt? - Welke aspecten (van de doelstelling) worden niet gehaald?
	5. Aan wat wijdt U de 'niet gehaalde' aspecten (van de doelstelling)?	
	6. Welke andere mogelijkheden ziet U om een antwoord te geven op dit type van behoefte?	- (Ander) afzonderlijk systeem? - Binnen het systeem van de verplichte verzekering?
	ANDERE STRATEGIEËN	
	7. Doen Uw leden in de praktijk beroep op andere (alternatieven) middelen (buiten het BSF) om de kosten die niet door de verplichte verzekering gedekt worden te vergoeden?	- Zo ja welke?

	8. In welke mate zijn deze alternatieven te veralgemenen en integreerbaar in een meer algemeen systeem?	
	PERTINENTIE VAN HET BSF	
	De interventiedomeinen betreffen: → *Tekst brochure tonen* □□de zeldzame indicaties □□de zeldzame ziekten □□de zeldzame ziekten die continue en complexe zorgen vereisen □□de verstrekkingen die innovatieve medische technieken zijn - met uitsluiting van geneesmiddelen □□de chronisch zieke kinderen □□ de in het buitenland verleende geneeskundige verstrekkingen 9. Welke andere elementen zouden eventueel door het BSF gedekt moeten worden? 10. Welke elementen zouden volgens U door dergelijk systeem niet gedekt moeten worden?	
	INFORMATIE OVER BSF	
	11. Hoe hebt U kennis gekregen van het bestaan en de functionering van het BSF? 12. Hoe wordt U geïnformeerd over evoluties (wijzigingen) in de regelgeving qua werking en tussenkomst van het BSF? a. Door wie? b. Welke soort informatie verkrijgt U (van wie) i. Informatie over de werking van het BSF ii. Informatie over regelgeving iii. Informatie over soorten tussenkomsten iv. Informatie over beslissingen BSF c. Hoe informeert U zichzelf? 13. Hoe beoordeelt U de kwaliteit van de beschikbare informatie rond het BSF? d. Op vlak toegankelijkheid e. Op vlak volledigheid	- Via welke media (internet, brochures, gidsen…)? - Vindt elke potentiële patiënt, die in aanmerking komt voor BSF tussenkomst de info gemakkelijk zonder hulp? - Informatie over de werking en criteria? - Informatie over de mogelijkheden van tussenkomst? - Informatie over mogelijkheden beroep tegen

	f. Op vlak bruikbaarheid – duidelijkheid 14. In welke mate worden/ werden jullie bij het opstellen – verspreiden van informatie rond het BSF betrokken? 15. Welke informatie rond het BSF verstrekken jullie aan de leden? 16. Welke informatie verstrekken jullie aan het BSF? 17. Welke informatie verstrekken jullie aan de ziekenfondsen? 18. Welke informatie verstrekken jullie aan de overheid?	beslissingen? - In welke mate is dit wenselijk/noodzakelijk? - Via welk communicatiekanaal?

	ERVARING VAN DE VERENIGING MET HET BSF / KENNIS OVER DE WERKING VAN HET BSF	
	19. Kan U beschrijven hoe het indienen van een aanvraag volgens U verloopt? vanaf de identificatie van een eventuele behoefte tot de uiteindelijke financiële tussenkomst door het BSF?	Wat is het traject van de aanvraag? - Wie stelt ze op? - Naar waar gaat de aanvraag? - Wie verstuurt ze? - Aan wie wordt ze verstuurd? - Welke zijn de volgende stappen?
	20. Van wie komt het initiële idee/voorstel om een aanvraag tot tussenkomst van het BSF in te dienen?	- Patiënt, ziekenfonds, arts, sociale dienst, andere? - Betrokkenheid ziekenfonds (lokaal niveau – niveau landsbond)?
	21. Wat kan U volgens U doen indien een aanvraag tot tussenkomst geweigerd wordt (komen jullie hierin tussen als ondersteuning van leden)? ▪ Op niveau van de patient? ▪ Op niveau van de vereniging?	(beroepsprocedure arbeidsrechtbank) Welke soort ondersteuning?
	22. In welke omstandigheden worden jullie bij het indienen van een aanvraag geconsulteerd of gevraagd ondersteuning te geven?	Hebben jullie hierover gegevens?
	23. In welke mate volgen jullie (globaal) het indienen – behandelen - van aanvragen tot tussenkomst door het BSF op?	

	24. Hoe beoordeelt U het geheel van de procedure?	Op gebied van: - duidelijkheid - complexiteit - administratieve belasting - bestede tijd - tijdsverloop - doelmatigheid - snelheid - communicatie - motivatie beslissing
	FUNCTIONERING VAN HET BSF	
	Criteria voor tussenkomst via BSF 25. Volgens welke criteria kan een dossier volgens U in aanmerking komen voor een tussenkomst vanuit het BSF? *! De respondent uitnodigen om de aangehaalde criteria te detailleren* *bv: «duur» → vanaf wanneer is iets duur?» - «voor bepaalde pathologie» → «welke pathologie?» …* Zie de lijst van criteria voor tussenkomst vanuit het BSF zoals door het BSF meegedeeld. *→ Grid tonen* 26. Wat is uw appreciatie voor elk van deze criteria in termen van relevantie (beantwoorden aan een reële nood), duidelijkheid en formulering? 27. Zijn deze criteria aangepast aan de noden van de patiënten? 27.1. Welke wel / niet? 27.2. Op welk vlak situeren zich de eventuele tekortkomingen? 27.3.In welke mate is er uniformiteit – contradictie in beslissingen? 27.4. Zijn er specifieke situaties waarvoor het BSF geen oplossing geeft?(welke) 28. In welke mate worden jullie als patiëntenvereniging geconsulteerd / betrokken bij wijzigingen in de regelgeving?	- Hebben jullie overleg met het BSF, het RIZIV, de overheden, de ziekenfondsen?

	29. Op welke vlakken hebben jullie zelf voorstellen tot aanpassingen van de regelgeving / criteria /interpretatie criteria geformuleerd?	- Gestructureerd?
	<u>Andere organisaties/initiatieven in binnen en buitenland die de belangen van personen met een zeldzame aandoening behartigen</u> 30. Welke organisaties / initiatieven kent U die de belangen van personen met een zeldzame ziekte behartigen? 31. Maakt uw organisatie deelt uit van deze initiatieven? 32. Op welk vlak leveren zij ondersteuning?	- Vb Fonds voor zeldzame ziekten en weesgeneesmiddelen beheerd door de KBS (www.weesziekten.be) - Sociale ondersteuning - Financiële ondersteuning - Wetenschappelijke informatie verstrekking
	<u>Systemen in het buitenland</u> 33. In welke mate hebben jullie kennis van hoe vergelijkbare situaties (die in België via het BSF geregeld worden) in het buitenland een oplossing krijgen? 34. Hoe werken deze systemen? 35. Kunnen ze als good practice gelden? 36. Hebben jullie contacten met patiëntenverenigingen in het buitenland?	Specifiek peilen naar Frankrijk, Nederland en Spanje? Contacten vragen
	<u>Algemene evaluatie van het BSF</u> 37. Welke positieve punten identificeert U in het bestaan en de werking van het BSF? 38. Welke negatieve punten identificeert U? 39. Welke wijzigingen of verbeteringen zou U voorstellen?	- In het systeem zoals het thans bestaat - Wijzigingen op het vlak van het systeem - Hoe kan het gebruik van het BSF verhoogd worden?

9.9 INTERVIEW GUIDE MEDICAL SPECIALISTS

9.9.1 Dutch version

Duurtijd (+ formulering van de vragen*)	Vragen	Hulp bij vragen
	INTRODUCTIE ONDERHOUD	
	- welkom + dank voor aanwezigheid - voorstelling + functies van moderator/verslaggever - doel van de studie - Toelichting wijze van selectie deelnemers - Verwachtingen ten opzichte van de deelnemer * Er zijn geen juiste of foute antwoorden: de visie van elke deelnemer is belangrijk - Toelating vragen om gesprek op te nemen (doel: minder nota's moeten nemen en antwoorden niet vervormd registreren) - confidentialiteit (o.m. nota's en opname die achteraf vernietigd wordt) - resultaten interviews komen in rapport dat beschikbaar zal zijn Januari 2010	**Objectieven van de studie** - perceptie nagaan van de nood om over een financieel vangnet voor belangrijke medische kosten die niet door de verplichte ziekteverzekering gedekt worden, te beschikken. - Andere strategieën en kanalen dan het BSF, gebruikt om deze soort uitgaven te dekken - Kennis over het BSF en de functionering ervan - pertinentie en efficiëntie van het BSF (inbegrepen tekorten op vlak van de tussenkomsten) - werking van het BSF en implicaties voor de respondent (procedure, criteria voor tussenkomst, beschikbare informatie, mogelijkheid tot aantekenen beroep, communicatieproces, positieve en negatieve elementen) - wijze waarop de respondenten gebruik maken van het BSF (eigen criteria, administratieve belasting,e.d.)

	INLEIDENDE VRAGEN / INFORMATIE OVER RESPONDENT	
	Specialisatie 1. Wat is uw profiel als geneesheer specialist?	- Basis specialisme? - Sub specialisme? - Werkzaam op welke dienst? - Eventuele specialisatie in bepaalde zeldzame ziekten? - Specialisatie naar specifieke patiëntengroepen (kinderen…?) - Private praktijk?
	Verhouding tot het BSF - context U (één of meerdere van uw patiënten) hebt gedurende de voorbije jaren verschillende aanvragen tot tussenkomst bij het BSF ingediend. 2.1. In globo, hoeveel aanvragen dient U per jaar in? 2.2. Op hoeveel patiënten hebben deze aanvragen betrekking? 2.3. Welk zijn de indicaties, pathologieën, aandoeningen of types van kosten waarvoor U aanvragen bij het BSF indient?	

	NOODZAAK AAN VANGNET /PERTINENTIE VAN BSF/ EFFICTIVITEIT VAN BSF	
	3. Waarom werd volgens U bovenop de verplichte verzekering het BSF als vangnet opgericht? De belangrijkste doelstelling van het BSF bestaat erin om financieel tussen te komen teneinde te voorkomen dat patiënten, in geval van een zeer ernstige medische toestand, aan zorgen zouden moeten verzaken, wanneer één of meerdere essentiële medische prestaties niet vergoed wordt(en) en deze prestatie(s) duur zijn (is). Deze (vraag tot) tussenkomst moet aan een aantal criteria beatwoorden.	
	4. In welke mate bereikt/haalt het BSF volgens U deze doelstelling?	- Welke aspecten (van de doelstelling) worden goed bereikt?
	5. Aan wat wijdt U de 'niet gehaalde' aspecten (van de doelstelling)?	- Welke aspecten (van de doelstelling) worden niet gehaald?

	6. Welke andere mogelijkheden ziet U om een antwoord te geven op dit type van behoefte?	- (ander) Afzonderlijk systeem? - Binnen het systeem van de verplichte verzekering?

	ANDERE STRATEGIËN	
	7. Doet U in de praktijk beroep op andere (alternatieven) middelen (buiten het BSF) om de kosten die niet door de verplichte verzekering gedekt worden te vergoeden? 8. In welke mate zijn deze alternatieven te veralgemenen en integreerbaar in een meer algemeen systeem?	
	RELEVANTIE VAN HET BSF	
	De interventiedomeinen betreffen: → *Tekst brochure tonen* □□de zeldzame indicaties □□de zeldzame ziekten □□de zeldzame ziekten die continue en complexe zorgen vereisen □□de verstrekkingen die innovatieve medische technieken zijn - met uitsluiting van geneesmiddelen □□de chronisch zieke kinderen □□ de in het buitenland verleende geneeskundige verstrekkingen 9. Welke andere elementen zouden eventueel door het BSF gedekt moeten worden? 10. Welke elementen zouden volgens U door dergelijk systeem niet gedekt moeten worden?	

	KENNIS VAN HET BESTAAN VAN HET BSF	
	11. Hoe hebt U kennis gekregen van het bestaan en de functionering van het BSF?	
	ERVARING VAN DE VERSTREKKER MET HET BSF / KENNIS OVER DE WERKING VAN HET BSF	
	12. Kan U beschrijven hoe het indienen van een aanvraag volgens U verloopt? vanaf de identificatie van een eventuele behoefte tot de uiteindelijke financiële tussenkomst door het BSF?	
	13. a. Volgens welke criteria kan een dossier volgens U in aanmerking komen voor een tussenkomst vanuit het BSF? *! De respondent uitnodigen om de aangehaalde criteria te detailleren* *bv : « duur » → « vanaf wanneer is iets duur ? » ; « voor bepaalde pathologiën » → «welke pathologie » …*	Wanneer en hoe beslist U om een aanvraag tot tussenkomst van het BSF voor te stellen?
	b. Van wie komt het initiële idee/voorstel om een aanvraag tot tussenkomst van het BSF in te dienen?	Patiënt/ arts /sociale dienst / andere
	c. In welke omstandigheden, indien dit zich voordoet, adviseert U een patiënt (of een arts) om GEEN aanvraag tot tussenkomst van het BSF in te dienen?	
	d. Wie neemt de finale beslissing om al dan niet een aanvraag tot tussenkomst in te dienen?	
	e. Wat gebeurt er concreet wanneer beslist werd om een aanvraag in te dienen?	Wat is het traject van de aanvraag? - Wie stelt ze op? - Naar waar gaan de aanvraag? - Wie verstuurt ze? - Aan wie wordt ze verstuurd? - Welke zijn de volgende stappen?
	f. In welke stappen komt U tussen en op welk vlak?	- Opstelling aanvraag? - verbetering / aanpassing aanvraag? - Bijkomende inlichtingen aan de sociale dienst van het ziekenfonds, de diensten van het BSF?

	.g- In welke mate wordt U, na indiening van een aanvraag, op de hoogte gehouden van het verder verloop van het (aanvraag) dossier?	- Wie informeert U? - Welke soort informatie verkrijgt U? - Motivering van de beslissing? - Wie wordt 'ook' geïnformeerd? - Door wie?
	14. Bij welke stappen in de procedure zou U, in voorkomend geval, meer betrokken willen zijn in het proces? Waarom? Hoe?	- informatie over de motivering van de beslissing? - wie zou dit moeten overnemen?
	15. Van welke elementen in de proces zou U, in voorkomend geval, en indien mogelijk ontlast willen worden ? Waarom? Hoe?	
	16. Wat is in het algemeen de 'uitkomst' van uw dossiers BSF?	
	17. Welke informatie met betrekking op de slaagkans van de aanvraag geeft U aan de patiënt? (voorafgaandelijk aan de beslissing BSF)?	
	18. Wat kan U volgens U doen indien een aanvraag tot tussenkomst geweigerd wordt?	
	19. Hoe wordt U geïnformeerd over evoluties (wijzigingen) in de regelgeving qua tussenkomst van het BSF?	Internet/ RIZV/ sociale dienst/ andere
	20. Hoe beoordeelt U de kwaliteit van de beschikbare informatie rond het BSF?	Duidelijkheid/ volledigheid/ andere
	21. Hoe beoordeelt U het geheel van de procedure?	Op gebied van: - duidelijkheid -complexiteit - administratieve belasting - bestede tijd - tijdsverloop - doelmatigheid - snelheid

		- communicatie - andere
	KENNIS OVER VERGELIJKBARE SYSTEMEN IN HET BUITENLAND	
	22. Volgens U, hoe worden de kosten die in België door het BSF ten laste worden genomen, in het buitenland vergoed?	(FR-ES -NL)
	FUNCTIONERING VAN HET BSF	
	Modaliteiten van functioneren van het BSF **Beslissingscriteria voor tussenkomst vanuit het BSF** Zie de lijst van criteria voor tussenkomst vanuit het BSF zoals door het BSF meegedeeld. → *Grid tonen* 23. Wat is uw appreciatie voor elk van deze criteria in termen van relevantie (beantwoordend aan een reële nood), duidelijkheid en formulering? **Algemene evaluatie van het BSF** 24. Welke positieve punten identificeert U in het bestaan en de werking van het BSF? 25. Welke negatieve punten identificeert U? 26. Welke wijzigingen of verbeteringen zou U voorstellen?	Uniformiteit: contradicties in de beslissingen? - In het systeem zoals het thans bestaat - Wijzigingen op het vlak van het systeem - Hoe kan het gebruik van het BSF verhoogd worden?

9.9.2 French version

Durée (+ formulation des questions*)	Questions	Aide aux questions
	INTRODUCTION À L'ENTRETIEN	
	- bienvenue + remerciement pour la présence - présentation + fonction des modérateur / rapporteur - but de l'étude - Explication de la sélection du participant - attentes vis-à-vis du participant * il n'y a pas de bonnes ou mauvaises réponses: la vision de chacun est importante - demander l'autorisation pour enregistrer (but : prendre moins de note et ne pas déformer les propos) - confidentialité (entre autres matériel d'enregistrement, notes) - résultats des entretiens : rapport disponible en janvier 2010	**Objectifs de l'étude** - perception du besoin de disposer d'un filet de sécurité financier pour les dépenses médicales onéreuses qui ne sont pas couvertes par l'assurance obligatoire - Autres stratégies et autres canaux utilisés que le FSS pour couvrir ce type de dépenses - Modes de connaissance du FSS et son fonctionnement - pertinence et efficacité du FSS (dont les manques dans les interventions) - fonctionnement du FSS et implications pour le répondant (procédure, critères d'intervention, information disponible, possibilité de faire appel, processus de communication, éléments positifs et négatifs) - manière dont les répondants utilisent le FSS (critères propres, charge administrative, etc.)

	QUESTIONS D'OUVERTURE / INFORMATIONS SUR LE REPONDANT	
	Spécialisation 1. Quel est votre profil en tant que médecin spécialiste ?	- spécialité générale? - sous spécialité? - travaille dans quel service? - spécialisation éventuelle dans certaines maladies rares? - spécialisation pour certaines populations (enfants, …)? - pratique privée?
	Rapport au FSS – contexte Vous (un ou plusieurs de vos patients) avez donc introduit pendant les années passées plusieurs demandes d'interventions au FSS. 2.1. Globalement, combien de demandes introduisez vous par an? 2.2. Ces demandes concernent combien de patients? 2.3. Quelles sont les indications, pathologies, affections ou type de coûts pour lesquelles vous introduisez des demandes au FSS ?	

	BESOIN D'UN FILET DE SECURITÉ /PERTINENCE DU FSS/ EFFICACITÉ DU FSS	
	3. D'après vous, pourquoi un système de filet de sécurité supplémentaire au système d'assurance obligatoire tel que le FSS a-t-il été crée ? Le FSS a pour objectif principal d'intervenir financièrement pour éviter qu'un patient, dans une situation médicale très grave, doive renoncer aux soins si une ou des prestation(s) médicale(s) essentielle(s) n'est ou ne sont pas remboursée(s) et est ou sont particulièrement chère(s). Cette intervention doit répondre à certains critères. 4. D'après vous, dans quelle mesure le FSS remplit-il cet objectif? 5. A quoi attribuez-vous ces « éléments manqués »? 6. En théorie, quelles autres possibilités voyez-vous pour répondre à ce type de besoin ?	- Quels sont les aspects qu'il remplit bien? - Quels sont les aspects non atteints? - Système à part ? - dans le système d'assurance obligatoire ?

	AUTRES STRATÉGIES	
	7. En pratique utilisez-vous d'autres moyens pour couvrir des coûts qui ne sont pas couverts par l'assurance obligatoire? 8. Dans quelle mesure ces stratégies sont –elles d'après vous généralisables, transposable dans un système plus global?	
	PERTINENCE DU FSS	
	Les domaines d'interventions concernent : → *MONTRER LE TEXTE DE LA BROCHURE* □□les indications rares □□les maladies rares □□les maladies rares qui nécessitent des soins continus et complexes □□les dispositifs médicaux et/ou prestations qui sont des techniques médicales innovantes - à l'exclusion des médicaments □□les enfants malades chroniques □□ les soins délivrés à l'étranger 9. Quels autres éléments devrait-il éventuellement couvrir? 10. Quels éléments devraient éventuellement selon vous ne pas être couverts par un tel système?	
	CONNAISSANCE DE L'EXISTENCE DU FSS	
	11. Comment avez-vous eu connaissance du FSS et de son fonctionnement?	

	EXPERIENCE DU PRESTATAIRE DU FSS / CONNAISSANCE DU FONCTIONNEMENT DU FSS	
	12. Racontez-moi comment se déroule de votre point de vue l'introduction d'une demande d'intervention depuis l'identification d'un éventuel besoin jusqu'à l'intervention financière ?	
	13. a- D'après vous, selon quels critères un dossier peut-il être éligible pour une intervention du FSS ? *! Inviter le répondant à détailler le contenu des critères qu'il cite par ex : « cher » → « à partir de quand est-ce cher ? » ; « pour certaines pathologies » → « quelle pathologie » …*	Quand et comment décidez-vous de proposer de demander une intervention au FSS ?
	b. De qui provient l'idée initiale de demander une intervention du FSS ?	Patient/ médecin / service social / autre
	c. Dans quelles circonstances, s'il en est, vous arrive-t-il de conseiller à un patient ou un médecin de ne pas introduire une demande d'intervention par le FSS ?	
	d. Qui prend la décision finale d'introduire une demande d'intervention ?	
	.e. Concrètement, une fois la décision prise de demander une intervention, que se passe-t-il?	Quel est le trajet de la demande?
	f. À quelle(s) étape(s) intervenez-vous et en quoi ?	- Qui la rédige? - Où va-t-elle? - Qui l'envoie? - A qui est-elle envoyée? - Quelles sont les étapes ensuite?
	.g. Après introduction de la demande, dans quelle mesure êtes-vous informé de la suite du dossier ?	- Rédaction? - Correction? - Information complémentaire auprès du service social? de la mutuelle? du FSS? - Qui informe? - Quel type d'information?

		- Motivation de la décision? - Qui d'autre est informé? - Par qui?
	14. Le cas échéant et si cela était possible, pour quelles étapes souhaiteriez-vous être plus impliqué dans le processus ? Pourquoi? Comment?	- Information sur la motivation de la décision ?
	15. Le cas échéant et si cela était possible de quels éléments souhaiteriez-vous être déchargé dans le processus? Pourquoi ? Comment ?	- Qui d'autre devrait s'en charger ?
	16. Quelle est en général l'issue de vos dossiers?	
	17. Quelle information relative au succès éventuel de la demande donnez-vous au patient préalablement à la décision?	Internet/ INAMI/ service social/ autre
	18. Si une demande d'intervention venait à être refusée, d'après vous, que pouvez-vous faire?	Clarté/ complétude/ autre
	19. Comment êtes-vous informé des évolutions des règles de remboursement du FSS?	En termes de : - clarté -complexité
	20. Comment jugez-vous la qualité de l'information disponible à propos du FSS?	- lourdeur administrative - temps à consacrer
	21. Comment jugez-vous l'ensemble de la procédure?	- délais - efficacité - rapidité - communication - autre
	CONNAISSANCE DE SYSTEMES A L'ETRANGER COMPARABLES AVEC LE FSS	
	22. D'après vous, comment les coûts qui sont remboursés en Belgique par le SSF, sont pris en charge à l'étranger?	(France - Espagne - Pays Bas)
	FONCTIONNEMENT DU FSS	
	lons maintenant des modalités de fonctionnement du FSS	

	<u>Critères de décisions pour intervention du FSS</u> Voici la liste des critères d'éligibilité annoncés par le FSS. → *MONTRER LA GRILLE* 23. Quelle est votre appréciation pour chacun d'entre eux en termes de <u>relevance</u> (répond à un besoin réel), de <u>clarté</u> et de <u>formulation</u>? **<u>Evaluation générale du FSS</u>** 24. Finalement, quels points positifs identifiez-vous dans l'existence et le fonctionnement du FSS? 25. Quels points négatifs identifiez-vous? 26. Quelles modifications ou améliorations suggèreriez vous?	Uniformité : contradictions dans les décisions? - Dans le système tel qu'il existe - Changement de système - Comment augmenter l'utilisation?

9.10 RESULTS OF THE INTERVIEWS WITH THE REPRESENTATIVES OF THE SOCIAL SERVICES

9.10.1 Knowledge on the existence of the SSF

The interviewed social workers have gained knowledge on the existence of the SSF trough the following channels: colleagues from the hospital's social service , the sickness funds, during their bachelor degree "social work", via the medical doctors of the hospital, via the cancer league, via a previous work experience in a sickness fund and finally via the SSF brochure. One notices the majority of social workers got this information from inside the hospital.

9.10.2 General information on SSF cases

The number of cases yearly submitted to the SSF by the social workers interviewed ranges between 40 (university hospital – big applicant) and 1 or 2 cases. This number can vary substantially over the years, depending on the patient's profiles, the evolution of the regulatory health insurance coverage and the criteria set by the SSF.

Respondents indicated that the number of cases has decreased substantially over the years due to inclusion (and reimbursement) of previous SSF cases into the regular health insurance system.

The social services stipulated they don't have the requisite medical knowledge to assess the SSF files on content. Only when they know that one or more SSF criteria aren't met, they inform the medical doctor of the possible non eligibility for reimbursement of the case by the SSF. This action can lead to the decision to stop the procedure on preparing or entering a SFF file.

None of the respondents could inform us on the exact number of applying medical doctors within their hospital (at hospital level) as the social services are not necessarily involved in the submission of all the SSF files (medical doctors can submit a SSF file on their own or patients can go directly to their local sickness fund to apply for SSF intervention). All respondents reported they have contact with a limited number of applying medical doctors, some respondents reported an exact number, ranging between 1 and 5. Since social workers are linked to medical services or hospital wards, this number does not represent the total number of medical doctors concerned. The data on SSF applications are not consolidated at hospital level. The global number of medical doctors involved in SSF cases clearly depends of the size of the hospital and the grade of specialization within the medical services and disciplines, but even in big university hospitals the global number of staff members implicated in SSF applications is limited. The following medical disciplines were linked to SSF cases: (pediatric) oncology, cardiology, (pediatric) neurology, metabolic diseases, nephrology, urology, hematology, gastroenterology and digestive surgery, radiotherapy and pulmonary diseases.

9.10.3 Need for a safety net/relevance of the SSF/effectiveness of the SSF

On the question "Why has a safety net as the SSF been established next to the regular health insurance system?" the respondents gave two major reasons which they see as major objectives for the SSF:

- To cover the costs of necessary and expensive medical treatments which are not (yet) included and reimbursed in the regular health insurance system. As such the SSF should be a real safety net and provide financial support to patients facing such situations. The objective mentioned is clearly based on the solidarity principle.
- To identify expensive medical treatments, drugs or medical devices which are not included in the regular health insurance system in order to gain knowledge on the use and the effectiveness of them and to document a later decision on eventual transfer to reimbursement by the regular health insurance system.

As a consequence, the SSF assures access to medical care through financial support (solidarity principle) in a kind of "waiting room" modus.

9.10.4 The SSF: meeting its objectives?

Some respondents reported the SSF achieves its objective of reimbursement of medical costs not covered by the compulsory health insurance at least for the SSF cases related to their hospital. The other respondents identified the following gaps in achieving the proposed objectives:

- Reimbursement is often limited to a percentage of the cost (varying from product to product- 50%, 60% and 70% were mentioned). The consequence is the patients still have to pay a sometimes very substantial amount themselves. Social services indicated that sickness funds are not always eager to submit a file to the SSF. No reasons were cited explaining this sense of hesitation.
- The throughput time (initiation to final decision) is sometimes long and demotivates patients/medical doctors to submit a file. It creates uncertainty about the outcome in a situation where the patient is confronted with a serious illness and is facing important medical expenses.
- The decision of the SSF is not send to the social service, only to the patient. The lack of a clear motivation of a negative decision to the social services is sometimes disappointing because social services have the objective to help their patients.
- Not all potential SSF cases actually result in submitted SSF files, as the SSF is not widely known among medical doctors or patients. As a consequence the SSF doesn't reach all her potential beneficiaries.
- For the same pathology and treatment, they noticed different reimbursement amounts in 2002. Similar SSF files can result in different reimbursement amounts. Similar cases result in different decisions.
- Some patients want certainty about the acceptance of their case before they give their consent to start up the treatment, due to a lack of financial resources. In these cases there is no invoice to submit to the SFF and decision times are long. If the decision is negative, patients as a consequence won't receive the optimal medical treatment.
- The premise of the SSF is not that the treating medical doctor, as an expert, is the right person to determine which therapy is the best for his/her particular patient and condition. The treating medical doctor is responsible that the most appropriate therapy is followed. His/her opinion and responsibility are central. The SSF doesn't start from a medical necessity of a particular treatment but rather from an administrative perspective and fixed limited budget.
- The term SSF "special solidarity fund" is confusing because the SSF doesn't intervene in all cases where there is no reimbursement in the regulatory health insurance system. Solidarity within the SSF is restricted and limited to very specific financial costs for very specific indications and pathologies, for which a whole set of criteria, must be met.
- Local sickness funds do not always treat the SSF applications objectively. Differences in treatment and advice from the advising doctor at local level are mentioned creating unequal treatment. Knowledge on the SSF at local sickness level differs substantially.
- The function of the SSF as a waiting room and a system for detection of new treatments or devices implicates the "waiting room" has to be emptied on time. Transfer of reimbursement from the SSF to the regular health insurance system takes too long.
- Patients are sometimes victims of conflicts of interests between the NIHDI and pharmaceutical companies. The fact there is no agreement on the price of a drug or a drug has is not registered in Belgium, or is not registered for a specific indication, may not have consequences for the individual patient or the hospital. Patients and hospitals are sometime seen as a hostage in such conflicts of interest.

- Patients living close to the Belgian border, have the possibility to get medical treatment and medication in the neighbour country and can as such have cost of treatments or medication reimbursed that is not reimbursed in Belgium.

The main reasons why the SSF doesn't reach her objectives mentioned were:

- The unfamiliarity with the SSF, as well within the group of medical doctors as in the group of social workers.
- The long duration time before a final decision is taken for some cases.
- Lack of knowledge concerning the exact definition of the SSF criteria and how these are interpreted in practice.
- The administrative focus of the SSF instead of a medical viewpoint.
- The fact drugs and medical devices are kept within the SSF reimbursement system for too long (several years) instead of transferring them to the regular health insurance system.
- The highly defined and very limited field of action of the SSF.

9.10.5 Alternatives for the SSF

On the question "What other options do you see to respond to this type of need?" the following answers were provided:

- The cancer fund (oncology focus): reimbursement of limited amounts
- Public centres for social welfare (OCMW/CPAS): focuses on persons with a particular social profile
- Private health insurances
- The social funds of the sickness funds
- Studies by pharmaceutical firms
- The fund for occupational diseases
- Organisation of charity event
- Parents associations, grants, charity institutions, non profit initiatives, …
- Caritas funds
- Support/social funds of the hospital
- MAB

All respondents indicated that none of the channels mentioned are a valid alternative to the SSF. These channels are "emergency solutions", no structural solutions responding to a need. The reimbursement of exceptional medical costs is seen as a responsibility to be covered by the health insurance system and can not rely on charity initiatives. The SSF is actually the only institution responding to the needs of patients who are confronted with high medical costs of necessary medical treatments, not reimbursed within the regular health insurance system.

9.10.6 The intervention domains of the SSF

9.10.6.1 Enlargement of the SSF intervention domains

On the question "Which other elements should to your opinion be covered by the SSF", respondents reported the following recommendations:

- The criterion on "experimental phase" needs to be widened.
- The criterion "rare disease and rare indication" limits the action field of the SSF. High medical expenses due to an exceptional medical treatment that is judged as appropriate by the treating medical doctor should be reimbursed regardless of the fact the disease or the indication is rare.
- The criterion on "vital function" is very restrictive. Hair transplantation for children is not a treatment of life-threatening nature but does offer a solution to a serious psychological/emotional problem.

- The category of "chronically ill children" should be extended to "all chronically ill patients".
- The off label use of drugs.
- The social and financial situation of the patient should be taken into account in the reimbursement amount. Otherwise the SSF doesn't cover what the term suggests.
- For some "social" categories no limitation in the reimbursement may be applied.
- The restriction to "one parent guidance" to other countries is problematic and emotionally unacceptable.

9.10.6.2 Limitation of the SSF intervention domains

On the question "Which elements should to your opinion be excluded from the current SSF system?" all respondents answered that there was no reason to reduce the existing intervention domains of the SSF. On the contrary, the suggestion was given to include current SSF cases as much as possible into the regular health insurance system and to look for solutions for all medical treatments not covered by the current system. The SSF has a role to play in the identification, the communication and formulation of suggestions for inclusion of treatments into the regular health system.

Five respondents explicitly suggested including the category of "chronically ill children" into the regular health insurance coverage. The inclusion of costs for medical treatment abroad was mentioned once.

9.10.7 Experience with the SSF/knowledge of the SSF procedure

The respondents described their view on the different steps and parties involved in the SSF procedure.

- Many respondents reported that the complete file (declaration on honor, scientific articles, medical information, invoice, …) is submitted to the local sickness fund of the patient. One interviewee reported that the SSF files are submitted directly to the national level (medical director) of the sickness funds. One social worker indicated the possibility to send the SSF file directly to the SSF.
- Among respondents knowing that files are submitted in a first step to the local sickness funds, some reported the internal transfer from the local sickness funds (advisory physician) to the national level (medical director). Consequently, many respondents believe that the local advisory physicians submit the files directly to the SSF and are not aware of the intermediate step within the sickness funds.
- It is not clear who the deciding authority is for SSF files: the advisory physician of the local sickness fund decides, or a committee of advisory physicians that meets once every two to three months; Some respondents refer also to a committee without further specification.
- Almost all respondents indicate that they are not involved or informed on the outcome and further follow-up of a SSF file. There is only feedback and communication with the local sickness funds when documents are missing. Contacts with the SSF or the sickness funds at national level (medical directors) don't exist.
- Social services are not informed about the final outcome of a SSF file unless the patient informs them that the SSF file is rejected and the patient is still confronted with the financial problem linked to the cost of the medical treatment.
- One respondent received the information from the sickness funds that the financial cost needs to exceed €2000 before a SSF reimbursement can be accepted.

- Many respondents are aware that an appeal procedure exists: Some of them know the "labour court" procedure. The procedure is perceived as complex, not really supported by the sickness funds. It increases the throughput time to a final decision and prolongs the uncertainty for the patients.

9.10.8 Communication and information dissemination

On the question "How do you get informed regarding evolutions/changes in the SSF regulation/procedure?" all respondents indicated that they are not systematically informed on these topics. Some respondents know the SSF brochure as source of information, one hospital has developed an internal brochure on the functioning of the SSF, and one hospital organizes internal training on the functioning of the SSF. Other "ad hoc" channels are: the cancer league, centers for social welfare, sickness funds and contacts with the NIHDI. None of the respondents indicated there were informed directly by the SSF on changes in regulation/procedure.

The quality of the available information about the SSF is judged as "too limited".

- Respondents indicate that the limited information in itself creates a huge quality problem. Direct feedback from the SSF regarding the files (decision and motivation) could improve the knowledge of the SSF enormously. The annual report is difficult to find and the information included is to general to realize "in depth knowledge" on the SSF. Two respondents suggested SSF decisions should be fully transparent and accessible. One respondent suggested the decisions of the labour courts on SSF cases should systematically be accessible.
- The SSF is described by three respondents as "surrounded by a haze of fog" or "a black box" because it acts very isolated, is not accessible and provides no transparency on its decisions. As additional problem, the remark was made several times that smaller sickness funds at their local level hardly know the procedures and functioning of the SSF. The comparison with the cancer fund has been made. This institution gives on an annual basis a presentation on their functioning and the most recent changes. A similar initiative from the SSF would improve the relation with the SSF and eliminate current unclearness.
- The available information is sufficient for some people: via the social services of the sickness funds we receive all necessary information. The SSF brochure has been described as handy and user friendly but it is not available at all campuses of the hospital.

9.10.9 Knowledge and appreciation of the SSF criteria

The criteria, necessary for approval of SSF cases that were reported spontaneously during the 12 interviews (without any support tool – grid) are listed below:

- Expensive : with the remarks that the term "expensive" is very depending on the individual financial situation of patients, rare diseases, serious diseases, beyond the experimental phase, not reimbursement within the regular health insurance system, essential care, appropriate treatment, exceptional situation, life threatening situation, no other reimbursement channels, added value must be proved, exceptional costs, exceptional high costs for drugs, administrative requirements including the submission of an invoice and declaration on honour, for chronically ill children more then € 650 of financial costs.
- As shown above, respondents could only report a limited number of criteria without the help of the criteria grid, often in more general terms. When asked for the definition of each criterion, respondents said they were not aware of the exact definition, used by the SSF to decide on the SSF cases.

The respondents were asked to formulate their appreciation of each criterion in terms of relevance (answering to a real need), clearness and formulation. This time the interviewer showed a grid in which all criteria were listed.

Table 19: evaluation of the SSF criteria by the social services

	Clearness			Relevant			Formulated sufficiently specific		
	Yes	Neutral	No	Yes	Neutral	No	Yes	Neutral	No
Rare indications	2		10	5	2	4	1		11
Rare diseases	4		8	7	1	4	2		10
Expensive	4	1	7	10	2	0	1	1	10
Related to a rare disease harming vital functions	7	2	3	9	1	2	6	2	4
Responding to an indication necessary on medical – social perspective	3	0	9	6	1	2	3	0	9
Scientific value and effectiveness	10	1	1	9	2	1	8	1	3
Passes the experimental stage	11	1	0	7	2	3	10	1	1
No therapeutic alternative in the regular health insurance system	11	0	1	10	2	0	10	0	2
Medical treatments abroad	11	1	0	11	1	0	10	2	0
Chronically ill children	9	1	2	5	3	4	6	1	5
Costs medical aids and treatments that are innovative medical techniques	8	1	3	8	2	2	5	1	6

NB: the occurrence of the answers are given as an indication but have no statistical signification. No inference can be done with these results due to the theoretical purposive sample, i.e. not representative in the statistical acceptation

9.10.9.1 Rare indication/rare disease

Both criteria on the rareness of the disease or the indication are definitely not clear to the social services interviewed. Since clearness of these criteria is poor, it is evident most of the respondents see the criteria as not specific enough.

For both criteria the relevance is not obvious at all for several people, as well for SSF intervention as for the rare disease One respondent did not express an opinion because the criterion was too unclear to judge the relevance.

Most of the interviewees stated the SFF reimbursement should not be limited to rare indications or rare diseases. They suggest expanding the action field of the SSF to special medical situations where the most appropriate medical treatment of the patient is not possible within the limits of the regular public health insurance regulation. (nomenclature codes). The fact a patient is not responding to a regulated drug or treatment for which prescription is possible under the regular health insurance regulation, the SSF should intervene in the costs and as such allow the prescription and the reimbursed use of the most adequate drug or treatment.

They find it is an individual right to have the most appropriate medical care, and if the regular health insurance system is not providing this, individual decisions on individual medical cases should fill this gap. There is a general consensus such situations must be assessed and decisions have to be taken on medical grounds only enabling the treating medical specialist to motivate his choice of treatment.

9.10.9.2 Expensive

The criterion expensive is not clear and not formulated sufficiently specific. For most of the respondents the criterion is relevant. A minimal cost as a criterion is largely accepted.

The unclearness is linked to the SSF decisions known and the lack of specific motivation of these decisions. It is not clear at all which specific internal criteria the SSF uses to determine the "expensiveness" of a drug, a medical device or a treatment. The specific internal criteria used by the SSF have to be known and to be fully transparent.

Most respondents stated that the meaning of 'expensive' depends from the individual social and financial situation of the patient. For some patients a yearly cost of 500 € is seen as expensive. Opinions on the question if the SSF reimbursement should be variable linked to the patients' income or financial situation differ. As the regular health insurance system does not link the right to reimbursement on individual income, most of them suggest the right to SSF reimbursement should not depend on personal income either. For the lowest income categories, the same protection as foreseen under the regular health insurance could be applied. (full reimbursement if the reimbursement for example is limited to a certain percentage, integration of the costs that are not reimbursed in the system of the MAB).

9.10.9.3 Vital functions

The criterion of vital function seems to be clear to respondents but it is not always judged as formulated sufficiently specific. One can conclude this criterion is not clear enough in its use in the SSF decision-making process. The relevancy of the criterion is widely accepted since seen relevant.

When asked to specify what the term vital means, we noticed confusion. Some see vital as related to vital organs and directly linked to a life threatening medical condition; others see vital as a condition were a patient cannot have a acceptable level of 'normal quality of life, mobility, functioning..).

9.10.9.4 Medical – social perspective

This criterion is definitely judged as unclear and not formulated specific enough. However it is seen by some as a relevant criterion. A few respondents did not express an opinion on relevance because of the unclearness of the criterion.

9.10.9.5 Scientific value and effectiveness:

This criterion is seen as clear, specific enough and relevant by most of the respondents. It is obvious to them the medical treatment or the drugs and medical devices or techniques have to be effective and must have a scientific value. Several respondents clarify this opinion by adding it is the treating medical specialist that has to argument his/her proposed treatment on scientific grounds, taking into account the individual medical situation of the patient concerned. Judgment on this criterion should be made by experts on the concrete disease.

9.10.9.6 Experimental stage

The criterion is clear, specific enough and relevant by some respondents. The remarks that were made on relevance are that in very special situations, it should be possible to use treatments or drugs for which classical studies have not been performed since it might be possible the disease is too rare to have these studies performed.

9.10.9.7 No therapeutic alternative in the regular health insurance system

The criterion is clear, specific enough and relevant. The only remarks on this criterion are one the alternative in the regular system has to be "equal" and guarantee the same medical outcome for the patient.

9.10.9.8 Medical treatment abroad

This category is clear, relevant and sufficiently specific to nearly all respondents. The non- acceptance of cost for a second accompanying parent has been mentioned as problematic in cases concerning children.

9.10.9.9 Chronically ill children

The criterion is clear and judged by some as formulated specific enough. Most comments regarding unclarity are related to the type of costs that are eligible for reimbursement. Some respondents see the category as relevant for the SSF. Others express the opinion it is questionable why this category is limited to children and why these costs cannot be integrated in the regular health insurance system.

9.10.9.10 Innovative medical techniques

The category is clear; however some respondents perceive the criterion as formulated not specific enough. The appropriateness of the prescribed assistive devices and medical treatments must be assessed correctly and the term innovative may not be equal to new. The treating medical doctor has to be enabled to make use of existing devices or treatments (medical techniques) that are not reimbursed under the regular health insurance system on specific non common indications or diseases.

9.10.10 The internal SSF procedure within the hospital

9.10.10.1 The initiator and final responsible for SSF applications

- The medical doctor was indicated in all interviews as the initiator of SSF files submission. The social service was sometimes mentioned as additional/complementary initiator. They intervene as an initiator when contacted by a patient on financial problems but also anticipate when they are aware of a situation (medical treatment) where an application for reimbursement by the SSF could be envisaged. The fact the social workers are operationally linked to the medical service of the hospital enhances the possibility for detection of such cases. Patients can play an active role in the initiation when they know the SSF or when communicating their financial problems with the hospital staff (nurses, medical doctors). The pharmacy department and invoice department were both mentioned twice as an initiator. They have a signal function when they detect the ordering of very expensive drugs or identify invoices for very expensive materials.
- The final decision to submit a file to the SSF is clearly a medical decision. In all interviews the treating medical doctor is indicated as the ultimate responsible and decider. Structural involvement of the social service has been mentioned; as well as the invoice department and the pharmacy department.
- In the hospitals where there is structural involvement of the social /financial or pharmacy department in the final decision, these departments advice the treating medical doctor on the SSF procedure. If they are convinced SSF criteria aren't met, the social services stop the procedure of submission to the SSF. In the other hospitals, the social services don't take up such a role since it's not their competency and responsibility or because the situation has not yet occurred.
- None of the social workers gives a specific prognosis on the changes of success for SSF files to patients in order not to create false expectations. This is a generalized hospital policy that counts for every intervention of the social services.

9.10.10.2 More or less involvement of the social services in the internal procedure

Many respondents ask for more involvement in SSF files. The request for more involvement is situated at two levels: the SSF and the hospital

- Towards the SSF, the main requests are related to having information regarding the final decision and more specifically the reasons/motivation why a file is approved/denied. Some interviewees would also appreciate more information on the status of a file (which step in the procedure? practical planning) and more transparency on SSF criteria and global decisions.
- Internally, at hospital level there is the aim to be more involved in the initiation of SSF files to avoid potential SSF applications not being detected and as a consequence prevent situations where patients don't get reimbursement, provide more support on content and finally more internal administrative coordination of SSF files.

Respondents don't want to decrease their involvement in SSF files, and formulated the following suggestions/remarks in order to increase efficiency and simplify the current procedure:

- Sending every SSF file via the local sickness fund of the patient and in a second step via the national level (medical director) towards the SSF has no added value. Sending SSF files directly to the SSF could simplify managing the files, create a single contact point, enhance transparency, improve equal treatment and decrease waiting times. Only two respondents assess the intervention of the sickness funds as positive to detect potential alternative funding sources, to check the completeness of the SSF file and have an advice formulated by the advisory physician.
- The global administrative workload (social service plus medical staff) is high. The following examples were identified during the interviews:
  - Administrative formalities for renewals although it is clear the patient will need the treatment for a long period;
  - Providing the same information for comparable cases resulting in duplication of information (and workload) the SSF already possesses;
  - Searching for information which is already at the disposal of sickness funds;
  - Searching for information which is already at the disposal of the SSF as part of the NIHDI (financial costs of material, drugs, …);
  - Obligation to provide the hospital's invoice for the material or the drug when entering a SSF file. This is a heavy administrative workload and can easily be postponed till after a positive decision on the application. For the financial department the search of invoices (invoice to the hospital) is rather complicated since drugs and medical devices are not bought piece by piece and invoices contain various products;
  - Non acceptance of a electronic signature on the medical file entered by the prescribing medical doctor;
  - For chronically ill children there are differences between the sickness funds with respect to the required level of detail. It is judged as unfeasible to provide all detailed information for all chronically ill children. The time spent on one case was calculated by a hospital as 20 working hours;
- The procedures for appealing a decision from the SFF are not adequate. Having to enter a petition to the Labour Court is a very heavy procedure. Sickness funds are not very keen on this procedure since in fact it's a contestation of a decision that was partially taken by the sickness fund itself or where the sickness fund at least was implicated. The patient has to advance the cost of a medical expertise (€1.500 to €2.000) and in practice it's not feasible his/her treating medical doctor is present at the moment of the expertise.

9.11 RESULTS OF THE INTERVIEWS WITH THE PATIENT ORGANISATIONS

9.11.1 Brief presentation of the interviewed patient organizations

9.11.1.1 DEBRA

Debra (Dystrophic Epidermolysis Bullosa Research Association) was founded in 1978 in the United Kingdom and as non-profit organization in Belgium in 1999.

EB (Epidermolysis Bullosa), a rare disease, can broadly be classified into three main groups: EB simplex, junctional EB and dystrophic EB. The exact number of EB patients in Belgium is not known, since no registry of the disease exists. The annual number of patients born with EB is limited and many of these children die at a young age. The database of Debra Belgium contains +/- 80 records of patients or families confronted with EB.

9.11.1.2 PH Belgium

The patient organization "pulmonary hypertension" Belgium was founded in 2001. In 2003, the organization joined the European Organization PHA Europe. The number of patients with PH (pulmonary hypertension) in Belgium is estimated around 350, the number of members of PH Belgium is situated around 150 people. PH is divided into 4 classes (I,II,III,IV) according to NYHA (New York Heart Association) classification. The severity of the disease increases in ascending order. Class IV patients get their treatment (drugs) usually via an electronically controlled pump system.

9.11.1.3 BOKS

BOKS (Belgian organization for children and adults with metabolic disorders) was founded in 1994. This umbrella organization represents 1300 diseases, which are individually too rare to generate interest and support from the wider public. BOKS represents about 550 households, of which 450 children are confronted with a metabolic disorder. 30 children are still waiting for the exact diagnosis of their disorder, 50 children died in the meantime. BOKS database includes around 1000 records.

9.11.1.4 NEMA

NEMA, the Flemish patient organization for neuromuscular diseases was founded in 1987. The members of this umbrella organization represent a wide variety of approximately 50 neuromuscular disorders. Nema welcomes not only patients but also parents, family members, friends, professionals or other relatives. Thanks to their close cooperation with the neuromuscular reference centers (NMRC's), there are able to keep their medical information up to date.

9.11.1.5 Radiorg.be

Radiorg.be (rare diseases organization Belgium), is founded in January 2008 and recognized by Eurordis as the Belgian Alliance for Rare diseases. Radiorg.be is an umbrella organization representing approximately 80 very specific rare disease patient organizations. Radiorg.be represents 6.5% of the Belgian population (65,000 people) of which approximately 75% are children. As the objectives of radiorg.be as an umbrella organization are quite different from the 4 other specific patient organizations on rare diseases, a separate paragraph is written on radiorg.be

9.11.2 Activities of the patient organizations

The objectives and activities listed below were mentioned by all the interviewed patient organizations:

- Support exchange of information regarding the rare disease towards patients in close collaboration with the medical profession (diagnosis, treatment and evolution of the disease);
- Support contacts between patients and their families and provision of moral support;
- Foster networking between patients, their families and the medical profession. The treatment of rare diseases is often provided in university hospitals or reference centres, which still act too isolated;
- Organise leisure activities for patients and their families;
- Create awareness for the specific needs of patients that have rare diseases, their relatives by putting their specific situation and problems in the spotlight.

All patient organizations interviewed have developed a website (www.debra-belgium.be; www.ph-vzw.be; www.boks.be; www.nema.be; www.radiorg.be) providing essential information on the rare diseases they cover. Additional information on contact persons, internal structure and scheduled activities as well as links to other parent organizations can be found.

The activities of the patient's organizations are largely based on the commitment of numerous volunteers, who are often patients themselves or relatives of patients. The deployment of paid staff, financed with own financial sources, is only feasible for the bigger organizations but remains relatively limited.

Larger patient organizations, which are often umbrella organizations have several working or diagnosis groups, each focusing on a specific disease or handling a specific theme.

All patient organizations indicate that it is hard to create awareness and get attention for the specific needs of patients with a rare disease. The small scale of these patient organizations and the limited number of patients they represent makes it hard to influence "policy makers". Joining of forces is essential.

Although patient organizations represent and group patients that have a specific rare disease, there is no "one to one relation" between a rare disease and the treatment that is necessary. For each of them, the medical treatment can differ substantially. As a consequence the financial costs related to their treatment also differ a lot. Each case is unique and needs to be handled this way.

9.11.3 Knowledge on the existence of the SSF

The respondents became aware of the existence of the SSF via the following channels:

- Information provided by the treating medical doctor.
- Other patient organisation.
- Information provided by employees (social workers) of recognised reference centres
- From the exchange of information between their patient members leading to knowledge on the fact some of them got financial support from the SSF while others, confronted with the same medical expenses, did not.
- Via a concrete case on feeding pumps, some years ago. As an individual consumer it was at that time impossible to purchase or rent this equipment. The hospital and sickness fund looked for solutions for this particular case and mentioned the SSF.

None of the interviewees could report us on the exact number of SSF files submitted by their patients/members on an annual basis. The patient organizations interviewed only give advice regarding SSF files when patients explicitly ask for their invention and/or support or when the patients report practical problems. Supporting SSF files is not seen as a core task. Secondly the Belgian "privacy legislation" and the "confidentiality of medical information" prevent that data from patients, submitting an SSF file can be gathered and released. As a consequence, patient organizations are unable to provide a consolidated and comprehensive overview of SSF files, the number of positive or negative decisions and the reasons behind.

Depending on the nature of the rare disease the necessary medical treatment can differ substantially.

- For EB (Epidermolysis Bullosa) patients, the majority of financial costs is caused by dressings that must be renewed on a daily basis (quality requirements are very high as the skin is very sensitive), needles, bandages, ointments and personal care products. Patients confronted with dystrophic EB have in general the highest financial costs. The medical doctor, initiating a SSF file is often a paediatrician as many EB patients are detected and treated in a neonatology department. The treatment plan is developed by a multi disciplinary team.
- For pulmonary hypertension patients the majority of financial costs is caused by very expensive drugs (Revatio® and Tracleer® in the past, Remodulin®, Flolan®, …). SSF files are mostly submitted by class IV pulmonary hypertension patients. The medical doctor, initiating a SSF file is often a specialist in pulmonary or cardiac diseases. Reimbursement of care equipment and disposables (needles, adhesive bandages…) is currently not foreseen and may represent significant amounts.
- For patients suffering from metabolic diseases, SSF applications can be related to a wide variety of necessary treatments: (orphan) drugs (off label use), feeding pumps, hart monitoring equipment, oxygen treatment. The medical doctor, initiating a SSF file is often a geneticist, biochemist, neurologist, (neuro) pediatrician, endocrinologist or medical specialist in metabolic diseases.
- For patients confronted with neuromuscular diseases, SSF applications can be related to a wide variety of necessary treatments. One type of SSF reimbursement received quite recently a lot of attention as the SSF decided to suspend the reimbursement of idebenone (Friedreich's ataxia) from March 2009 onwards. The medical doctor, initiating a SSF file is often a (paediatric) neurologist.

9.11.4 Need for safety net

For the patient organizations interviewed, the SSF is an essential safety net for patients with rare diseases, covering the financial costs of treatments, which are necessary and not yet reimbursed through the regular health insurance system.

The SSF is perceived as a good initiative as it is the only way to obtain some kind of reimbursement.

For the patient organizations it is crucial that this financial support is provided now, but also guaranteed in the future. It is less important by which channel or institute this support is provided (via the specific SSF, via the regular health insurance system or other channels) as long as a compensation and reimbursement for these medical costs is foreseen.

The question "why should we exclude a patient from receiving an essential but expensive treatment?" was raised during the interviews. Each patient has the right to receive the 'necessary'/'optimal' treatment even when financial costs are high. Accessibility from a financial viewpoint needs to be guaranteed, through the solidarity principle.

Respondents indicate that rare diseases mostly are genetically determined. As a consequence the patient has no impact on getting ill and can do nothing to avoid the disease. Patients just are confronted with the rare disease. One respondent adds that many other diseases are influenced by people's lifestyle (smoking - lung cancer, alcohol abuse – liver diseases...) but such 'unhealthy behavior' is never used as a criterion for reimbursement within the regular health insurance system.

9.11.5 The SSF: meeting its objectives?

On the question "to what extent does the SSF reaches its objectives" all respondents indicated that the SSF is crucial for them and even when the SSF achieves its objectives only partially, the SSF is the ultimate and only solution for many patients to have medical costs reimbursed. The respondents identified the following gaps in achieving the proposed objectives:

- Not all potential SSF cases actually result in submitted SSF applications, as the SSF is not widely known among medical doctors or patients. As a consequence the SSF doesn't reach all her potential beneficiaries.
- One patient organization indicated that even medical doctors, treating rare diseases on a daily basis don't have the reflex or automatism to consider an SSF application.
- The administrative workload for medical doctors is high and submitting SSF files is not their primarily focus. If a patient is aware of the existence of the SSF, he/she can explicitly ask for the submission of a SSF file.
- Patients or parents have other worries when they are confronted with the diagnosis and treatment of a rare disease. The start-up of an optimal treatment is definitely their first priority. The financial consequences related to the treatment only show up in a second stage.
- The remark that patients have to find their way themselves in the multitude of existing support initiatives is not only applicable to the SSF. Respondents indicated that it is very difficult to know all the existing provisions since these are much disseminated. There is no single point of contact. Luckily various initiatives exist but finding them is a real nightmare.
- Even when patients know the SSF, they can decide not to submit a SSF file for different reasons:
  - The cumulative criteria listed in the SSF brochure create the expectation that only very exceptional cases are approved.
  - Patients expect that given the name "social solidarity fund" their financial situation will be taken into account in the final SSF decision and decide therefore not to submit a SSF application (don't want to put their financial situation on the table or expect a negative decision when their financial resources are not low)
  - Some patients don't submit a SSF application as they judge for themselves they are able to carry the financial cost of the treatment.
- Respondents indicate that a "grey zone" exists regarding which treatments have a potential chance of being reimbursed by the SSF. The following examples were given: reimbursement of essential physiotherapy more than twice / day, vitamins, the need for another drug than the one that is registered as the orphan drug related to the patients' individual indication or disease.
- Knowledge on the SSF at local sickness level differs substantially. It is not realistic to expect that all staff and advisory physicians of the local sickness funds know the existence and functioning of the SSF in detail and have medical knowledge on all rare diseases and their treatments.
- The term SSF "special solidarity fund" doesn't cover what the term suggests as the SSF doesn't intervene in all cases where there is no reimbursement in the regulatory health insurance system. Solidarity within

the SSF is restricted and limited to very specific financial costs for very specific indications and pathologies, for which a whole set of criteria, must be met. The SSF covers only a specific part of problematic situations.

- Patients are sometimes victims of conflicts of interest between the NIHDI and the pharmaceutical or medical device companies. The fact there is no agreement on the price of a drug or a drug is not registered in Belgium, or is not registered for a specific indication (off label use) may not have consequences for the individual patient, for whom the treatment is essential.

The main reasons why the SSF doesn't reach its objectives mentioned were:

- The unfamiliarity with the SSF, as well within the group of medical doctors as in the group of patients affected by a rare disease.
- Lack of knowledge regarding the treatments and costs that could be reimbursed via the SSF.
- The administrative workload for the medical doctors.
- The highly defined and very limited field of action of the SSF.

9.11.6 Alternatives for the SSF

On the question "What other options do you see to respond to this type of need?" the following answers were provided.

- Private health insurances;
- Respondents indicate that there are substantial differences between insurance companies – adhering is often impossible when the patient has a rare disease already - rare diseases are sometimes excluded - a maximum amount of costs is foreseen and reimbursement is limited to hospitalization;
- Public centers for social welfare (OCMW / CPAS);
- Additional hospitalization insurance offered by the sickness funds;
- VAPH: Flemish Agency for Persons with Disabilities (Bijzondere Bijstandcommissie/special assistance committee/BBC) for medical aids. The names BBC (bijzondere bijstand commissie) and BSF (bijzonder solidariteitsfonds) are confusing for patients, sometimes thinking these are the same initiatives.
- The social funds of the hospitals or the sickness funds on very exceptional circumstances
- The pharmaceutical companies (providing the medication for free to patients)

All respondents indicated that none of the channels mentioned are a valid alternative to the SSF as they are restricted to the hospitalization phase, limited in action field (medical aids), have limited financial resources and/or only applicable in exceptional circumstances.

9.11.7 The interventions domains of the SSF

9.11.7.1 Enlargement of the SSF intervention domains

On the question "Which other elements should to your opinion be covered by the SSF?" respondents reported the following recommendations:

- Reimbursement of personal care equipment and disposables (needles, adhesive bandages, soap, disinfectant, catheters used for injection of medication via pumps) is currently not foreseen and can represent significant expenses.
- Technical aids (aspirator, hart monitoring equipment) are necessary for a very limited number of our patients (exceptions within the broader group of our rare disease) but no structural support (reimbursement) is foreseen.
- Side effects of drugs can have positive impact for certain metabolic diseases (off label use).
- The terms "scientifically proven" and "prescribed by the treating medical doctor for an individual patient as the most optimal treatment" are two different aspects. For rare diseases, it is sometimes impossible or very complex to carry out scientific studies. The patient organisations draw attention to the fact that the number of patients affected by a rare disease is by definition limited (too small sample for scientific studies) and secondly that the treating medical doctor often don't want to take the risk that a patient would not receive the required medication (division of patients into 2 groups with one group receiving the administered medication and the other group receiving a placebo). The SSF should rely more on the expertise of the treating medical doctor, who is an expert.

9.11.7.2 Limitation of the SSF intervention domains

On the question "Which elements should, to your opinion, be excluded from the current SSF system?" none of respondents judged the current intervention domains of the SSF as redundant. On the contrary, patient organizations would welcome the enlargement of current intervention domains (see recommendations above).

Besides the intervention domains of the SSF, patient organizations would welcome solutions for all medical treatments not covered within the regular health insurance system.

9.11.8 Experience with the SSF / knowledge of the SSF procedure

Two procedures exist within the SSF. The decentralized procedure (complete delegation of SSF files to the sickness funds) is applicable for EB patients represented by Debra Belgium. The centralized procedure (submission of SSF files to the sickness funds first at local level, afterwards at national level and finally towards the SSF) is applicable for the 3 other patient organizations.

9.11.8.1 The decentralized procedure

The text below is based on the interview with Debra Belgium (2 interviewees). They described their view on the different steps and parties involved in the submission of a SSF application.

For SSF files related to Epidermolysis Bullosa (EB) patients, a specific procedure has been established. All SSF files related to EB patients, are delegated directly from the SSF to the sickness funds, meaning that SSF files are submitted, treated decentralized, outside the SSF. For these cases a final decision is taken, not by the SSF, but by the medical advisor of the local sickness fund. This decentralized procedure came into force in 2001. The main objective was to shorten the throughput time of an application. The College of medical directors had the task to further stipulate and decide which rare diseases and treatments could fall under this specific procedure.

Patients with EB immediately fell under this decentralized procedure as they were explicitly mentioned in the Royal Decree.

- This decentralized procedure (delegation) of EB files towards the sickness funds is positively assessed by the respondent(s) in comparison with the central procedure of the past. However, a risk of unequal treatment of patients exists.
- The different stages of the central procedure were perceived as problematic mainly at sickness fund level. Files were copied a lot and each intermediate level added its comments and suggestions with at the end a completely illegible copy and a very long throughput time. For patients, after such a long time lapse, it was unclear which costs were accepted for reimbursement and which were rejected.
- The decentralised procedure handled by the sickness funds has greatly reduced the throughput time.
- The decentralised procedure has one disadvantage. The respondent(s) indicate that there are differences between the requirements set by the different sickness funds. For example, some sickness funds require the adjustment of the treatment plan when a new product (eg new dressing) is used. Other sickness funds don't request this adaptation. There are also regional differences within the same sickness fund. Although the problem is perceived as rather limited by the patient organisation, patients don't understand these differences and expect equal treatment in regard to their peers. Till now the patient organisation did not make an inventory of these differences and did not inform the sickness funds at the national level of these differences.
- The ideal scenario would be a short procedure with uniform decisions. The interviewees indicate that it is not realistic to expect from local employees and/or advisory physicians of the sickness funds to be aware of all developments (including new materials) regarding EB. Centralisation of SSF files within the sickness funds would increase medical knowledge on EB, increase knowledge regarding new developments and products and contribute to uniform decisions. An additional advantage is the creation of a single point of contact within each sickness fund for all questions related to EB SSF files.
- Debra Belgium has always supported the vision to create an EB centre with as ultimate aim the clustering of medical knowledge regarding EB and the realisation of structural psycho-social support for EB patients and their relatives/parents. This EB centre is not yet reality although one university hospital takes up this role more and more in practice. In this hospital the social nurse takes the initiative to inform parents of newborns with EB on the existence of the SSF and supports the submission of the SSF files. Despite the fact that a large part of the newborns with EB are treated in this hospital, and thus find their way to the SSF, Debra has no view on the number of children being born with EB in other hospitals and which part of them is being informed on the SSF. Centralisation of EB patients and as a consequence acting on a "bigger" scale and combining resources would ease a more structural approach regarding patient counselling not only during the hospital phase but also after discharge.
- The more patients with rare diseases are clustered the more they will be informed on all kinds of existing support options including the SSF and the more the SSF will reach all her potential beneficiaries.

9.11.8.2 The centralized procedure

The text below is based on the interviews with 3 patient organizations (PH pulmonary hypertension, BOKS metabolic diseases and NEMA neuromuscular diseases):

- SSF files are mostly initiated by the treating medical doctor. In Belgium, there are 8 recognised reference centres for inherited metabolic disorders and 6 recognised reference centres for neuromuscular diseases. For pulmonary hypertension the majority of patients are treated in 3 hospitals. The social services of the hospitals, the social services of the sickness funds or the patients themselves if they are aware of the existence of the SSF can also play an active role in this initiation phase.
  - As indicated before the remark was made that even medical doctors treating rare diseases on a daily basis don't have the automatism to initiate a SSF application.
  - The administrative workload of a SSF file for the treating medical doctor was reported as heavy.
- Two patients organisations know that SSF files are submitted to the local sickness funds (advisory physician), afterwards transferred to the national level (medical director) and in a third phase submitted to the SSF. One patient organisation is not aware of these intermediate steps
  - It is impossible and unrealistic to expect from the employees and/or advisory physicians of the local sickness funds, to have an in depth knowledge on the treatment of all rare diseases, on the legal context and on the functioning of the SSF. As a consequence they can not assure optimal and up-to-date advice to their members, who are potential beneficiaries of the SSF. Centralization of knowledge within the sickness funds could offer a solution for this problem.
  - The added value of the local sickness funds is questioned. Respondents indicate that local sickness funds provide support in completing the administrative formalities and they check if the SSF file is complete.
  - The 3 step procedure is complex and patient organizations indicate that the majority of their members / patients don't know these different stages. This statement is further supported by the finding that the majority of their members even don't know the SSF as such.
  - Patients are not aware of the complex administrative procedure and it should not be their concern. When the diagnosis of a rare disease is made, the patient or parents start a hellish quest for support ranging from psychological support, finding a suitable school to the search for medical devices. All these aspects seem evident or negligible when seen isolated, but the quest for all aspects together is time consuming, complex and frustrating. Many supporting initiatives exist but the gateway to reach them seems to be closed. The creation of a single point of contact, where knowledge about all existing support initiatives, including the SSF, is centralized would mean a world of difference for the patients and their parents. According to the respondents the following institutions could play an active role in creating awareness for this problem and support the creation of the suggested initiative: Radiorg.be as the national umbrella patient organization for rare diseases, the 3 regional umbrella patients organizations (Flemish patient platform/Vlaams patiënten platform, LUSS Walloon patient organization/Ligue des usagers des services de Sante", "Patientenrat & treff" patient organization in the German speaking region) and organizations such as "Kind en Gezin" as rare diseases are often detected at a young age.
- The SSF is not very well known amongst patients

  - It is not clear who the deciding authority is at SSF level. For one patient organization the difference between the SSF and the commission on orphan drugs is not clear.
  - Patients don't know the SSF and are certainly not aware the SSF is a part of the NIHDI.
- The percentage of SSF applications that are completely rejected is relatively low (no precise indication of a %). If so, it puts the patient in a very problematic situation.
- The patient organisations recognise and appreciate the role the SSF plays in detecting expensive medical treatments, drugs or medical devices not covered within the regular health insurance system and the actions undertaken to include some of these into the regular health insurance coverage. Patient organisations gave examples of previous reimbursements by the SSF, that are now included in the regular health insurance system.
- The throughput time of SSF files has decreased over the years but can still be significant from a patient perspective.
- Patients are informed about the outcome of their SSF application, but often this information is unclear and in some cases outdated. If medication is essential for a patient, the treatment needs to start urgently. As a consequence medication has been administered before a final SSF decision is taken. For very expensive drugs an emergency procedure must be possible to avoid patients being confronted with a major financial risk.
- The SSF is perceived as a very isolated department since no direct contact between the SSF and the patients is allowed. One respondent stated that it's strange that such complex and individual SSF cases are completely evaluated on paper. Patients are never asked to appear in person and a patient can never explain or illustrate his/her SSF application from a patient perspective.

9.11.9 Knowledge and appreciation of the SSF criteria

The criteria, necessary for approval of SSF cases that were reported spontaneously during the 4 interviews (without any support tool – grid) are listed below:

- Rare disease, expensive remarks by all respondents that the definition of "expensive" is not known, life threatening situation, complex, beyond the experimental phase, added value must be proved, prescribed by a medical doctor specialist, not reimbursement via the regular health insurance system
- As shown above, respondents reported a limited number of criteria without the help of the criteria grid, often in more general terms. When asked for the definition of each criterion, respondents said they were not aware of the exact definition, used by the SSF to decide on the SSF cases. Only for the criterion rare disease, two respondents immediately referred to the proportion of 5 persons affected on a population of 100.000 persons.

The respondents were asked to formulate their appreciation of each criterion in terms of relevance (answering to a real need), clearness and formulation. This time the interviewer named the different criteria or showed a grid in which the criteria were listed. The respondents did mostly not understand the meaning of the criteria and therefore did not answer the notions of relevance and formulation. Patient organizations hardly know the criteria and perceive them as medical issues that have to be judged by the physician judge the cases according to the criteria.

Table 20: evaluation of the SSF criteria by the patient organisations

	Clear criterion	Unclear criterion
Rare diseases		
Rare indication		
Expensive		
Harming vital functions		
No therapeutic alternative in the regular health insurance system		
Scientific value and effectiveness		
Medical aids and treatments that are innovative medical techniques		
Chronically ill children		
Medical treatment abroad		

9.11.9.1 Rare diseases

Some patient organizations made the remark that this definition is not leading to any restriction for their members but the strict use of this definition by the SSF confirms the limited action field of the SSF. Many diseases don't fit into this strict definition and for those patients no structural solution is available when they are faced with high financial costs for a necessary treatment that is not reimbursed within the regular health insurance coverage.

9.11.9.2 Rare indications

Respondents perceive it as an enlargement of the strict "rare disease" definition.

9.11.9.3 Expensive

For the respondents it is not clear if a minimum amount is used by the SSF and how they calculate the cost (on an annual basis?).

The remark was made by three patient organizations that even when the separate costs of treatments are limited, the cumulative cost can be very high based on the recurrent characteristics of the treatment. Costs not reimbursed by SSF can appear as acceptable (personal care products, travel costs, …) but in many cases patients that are affected by a rare disease have less financial resources at their disposal.

The remark was made that 'expensive' is a very depending on the individual patient situation. Five hundred euro can be 'very expensive' for one patient and for another patient it might be easily supportable.

One patient organization supported the idea of adjustment of the level of reimbursement by the SSF depending on the financial situation of the patient, where a minimum reimbursement level should be guaranteed for everybody. The other patient organizations didn't support this idea of different reimbursement levels (depending on the financial situation) as patients are confronted with a rare disease and they didn't choose for this situation. Consequently reimbursement of the medical cost needs to be guaranteed.

Patients with limited financial resources should be encouraged to get the best treatment that is available. Even if full reimbursement is foreseen, these patients have often less access to optimal care. A more active approach in order to reach these patients is required. For these people a third payer system should be established. One respondent made the remark that taking the financial situation into account could also create a threshold and inhibit access to care. Patients are reluctant to have their financial situation to be put on the table.

9.11.9.4 Harming the vital functions

This criterion is not clear as during the interviews different interpretations and questions showed up: Can we interpret vital functions as important functional limitations or does it refer to a more life threatening situation (stopping of breathing, cardiac arrest, ...) or does it refer to the harm of important organs (hart, lung, kidneys, …)?

9.11.9.5 No therapeutic alternative in the regular health insurance system:

The remark was made that the alternative needs to be an equivalent solution

9.11.9.6 Scientific value and effectiveness

For respondents it is clear that the treating medical doctor must be convinced of the scientific value for his/her particular patient. The lack of scientific studies and prove can't be used as an exclusion criterion if the feasibility of such a study is not realistic.

9.11.9.7 Costs of medical aids and treatments that are innovative medical techniques

The criterion is judged as unclear

9.11.9.8 Chronically ill children

The category is very relevant but it is not clear what the exact definition of chronic is: ill during a minimum timeframe (month, 6 months, 1 year, …) or a disease we can not completely heal

9.11.9.9 Medical treatments abroad

For respondents it is not clear if there is a strict limitation to the medical treatment or if additional costs (care costs) are included

9.11.10 Communication and information dissemination

The text below is based on the interviews with 4 patient organizations (Debra Epidermolysis Bullosa - PH pulmonary hypertension, BOKS metabolic diseases and NEMA neuromuscular diseases)

- Some patient organisations know or expect an appeal procedure to exist. No respondent is aware of the "labour court" appeal procedure.
- On the question "How do you get informed regarding evolutions / changes in the SSF regulation / procedure?" all patient organisations indicated that they are not systematically informed on these topics. The following information channels were named to used to update information:
  - Internet search
  - SSF brochure
  - Ad hoc contacts with other parents
  - Complaints of members of the patient organization
  - Direct contact with employees of the NIHDI (not SSF)
  - Direct contact with the medical director of the sickness funds (national level)

None of the respondents mentioned the SSF itself as a structural information source. The SSF did not contact, explain or present its intervention domains or activities to the patient organizations (rare diseases) interviewed.

- The patient organizations were never asked by the SSF to act as information disseminators towards their members. By using this communication channel the SSF could create awareness on its existence and its functioning. All patient organisations indicated they are prepared to disseminate SSF information to their members. In the future radiorg.be could act as a single point of contact for the SSF when information dissemination to patient organisations for rare diseases or their members is required or judged as usefully.
- The patient organisations interviewed indicate they did not provide structural information to the SSF either. Most patient organisations have individual ad hoc contacts with employees of the NIHDI but not with the SSF directly.

- The patient organisations don't systematically provide information towards the sickness funds. Ad hoc contacts are always linked to specific SSF cases where further clarification is needed. Patient organisations only intervene when they receive a specific request from a member.
- Some organisations indicate that they maintain(ed) extensive contacts with politicians to get attention for the specific situation of the members they represent. These actions resulted in numerous parliamentary questions. They also gave the opportunity to have some specific individual patient situations put in the spotlight. These also raised media attention and created and more public awareness for rare diseases. Structural contacts directly related to the SSF with the ministry of health are seldom.

9.12 RESULTS FROM THE INTERVIEWS WITH THE PRESCRIBING PHYSICIANS

9.12.1 Knowledge on the existence of the SSF

Most doctors learned of the existence of the SSF from their practice. None of the interviewees got preliminary information directly from the SSF itself. The following channels were mentioned: from the hospital's social service, the sickness funds or its medical advisor, from colleagues, via a pharmaceutical company. Finally one interviewee could not recall how he first heard about the existence of the SSF.

9.12.2 General information on SSF cases

The number of cases submitted to the SSF is variable. Five of the respondents have a very limited number of SSF cases (1 to 2 on an annual basis) at the moment. Most of them had more cases in the past. The diminishment of the number of cases originates from drugs or medical devices that were transferred to the regular health insurance system or drugs that have been accepted as orphan drugs. Five other respondents have between 5 and 10 SSF files a year, 2 have between 10 and 15 cases and one has about 20 SSF files a year.

Most of the medical doctors keep no separate track of SSF files and have no consolidated data on SSF applications. For some medical doctors interviewed, the social worker or the social nurse takes charge of the administrative process of preparing a SSF application file. These social workers or social nurses are part of a centralized social service at hospital following some doctors, this role is taken over by his secretary, others get the support from the hospitals administration or handle the files completely on their own. Overall the doctors have little contact with their colleagues from other disciplines in the hospital on sharing experience with SSF procedures. Most physicians confer occasionally with colleagues from other hospitals on SSF experiences.

9.12.3 Need for a safety net/relevance of the SSF/effectiveness of the SSF

On the question why the SSF has been established as a safety net next to the regular health insurance system, the respondents gave three major reasons which they also see as three major objectives for the SSF:

- To allow new medical treatments not covered by the regular health insurance system and to reimburse the costs of these. As such the reimbursement by the SSF is a way to have solidarity at society level for exceptional individual medical cost of patients that have serious diseases. The SSF is perceived merely as a technique to limit the costs of new innovative medical treatments or drugs.
- A way to facilitate the use of new medical treatments, medical devices and drugs for patient care before all procedures to integrate them in the regular health insurance are completed.
- A kind of waiting room for new techniques and drugs that allows public health authorities to get more understanding on relevance and effectiveness.

For most respondents the existence of the SSF as such is positive. They see it as one of the only possibilities to have treatments, drugs or medical devices that are not reimbursed under the regular health insurance system, accessible for patient use. In this perspective many respondent expressed the opinion there is a need for the SSF (or another system with similar objectives) as a complement to the general insurance system. All of them are convinced it is impossible to have every situation regulated in the general health insurance system.

9.12.4 The SSF: meeting its objectives?

Most respondents judge the achievement of objectives by the SSF from their own direct experience. Some of them had a positive experience regarding the reimbursement of the drugs or medical devices for which they introduced an application. A solution for the practical issue they were facing was provided: the SSF reimbursed the costs. However the difficulties to obtain that reimbursement are judged as rather important.

The other respondents got mixed feelings regarding their experience with the SSF.

The SSF is sometimes perceived as an adequate and fully satisfying solution for the introduction of the innovative medical treatment he learned abroad (in the US) and wanted to apply on his patients in Belgium. The acceptance by the SSF of this new technique (colon stent) took about 6 months and since a few years the technique is part of the general health insurance system. In this case the SSF fully met the objectives on reimbursement of new innovative techniques as well as the objective to function as a kind of waiting room for general coverage of the costs of new medical treatments.

In another mentioned, the drug is already reimbursed by the SSF for a very long period (13 years), causing a side effect regarding the price of this drug. The price has never been subject of negotiation with the pharmaceutical company and as a consequence there has never been any 'regulation' on the price. The conclusion is, there is a solution for the patient (reimbursement) but it's a bad situation for the public health insurance system. For this type of reimbursed drugs the objective of the SSF as a "temporary" waiting room is clearly not met.

Respondents identified the following gaps in achieving the SSF objectives:

- Reimbursement is often limited to a percentage of the cost leaving a non solved problem for the hospital and the patient. Theoretically the patient is accountable for the part that was not reimbursed by the SSF. The hospital gets an invoice from the pharmaceutical firm for the full cost. The invoice is addressed to the patient but often the patient is not asked to pay the amount (the part that was not reimbursed by the SSF) till the moment the NIHDI and the pharmaceutical company reach an agreement on a fixed price and the drug is transferred to the regular health insurance system. In the meantime the hospital is in a difficult situation towards the pharmaceutical company (open invoice that can reach very important levels) and the patient still is in a very uncertain position not knowing what the final outcome of the SSF application will be. Some respondents see this situation as very problematic and see the patient and the hospital as a kind of hostage of both other parties. The NIHDI is judged as negligent towards as well the patient as to the hospital. The position of the prescribing doctor towards the hospital and the patient is not comfortable either.
- Patients are the ultimate victims of conflicts of interests between the NIHDI and pharmaceutical companies. The fact the procedure for acceptance (registration) of a new drug is complicated and time consuming results in non availability of new and necessary drugs for specific treatments. This is not acceptable from a medical point of view. Belgium is seen as one of the countries were the introduction of new drugs last the longest in Western Europe.

- The time to obtain a decision by the SSF (initiation to final decision) is in general too long. For some treatments it is ethically or medically not acceptable to postpone the start of the treatment till after having a decision. In some cases the patient is not prepared to take a risk of non acceptance by the SSF, leading to a real dilemma and leading to non adequate medical care. Medical needs are not fulfilled and no optimal medical treatment is provided. One respondent mentioned a medical treatment had to be interrupted for a 3 months period because a decision lasted too long. In this specific case the treating physician had to substitute the drug by another less effective one.
- The outcome of an SSF application is not predictable. Several respondents said they obtained different decisions on similar cases, or new colleagues that had a different outcome on quasi identical cases.
- Decisions are not motivated. The only information that is disseminated is that the application is not accepted for reimbursement since a criterion is not met (pure administrative motivation based on the SSF regulation). No specific medical motivation is provided. For all respondents the motivation is clearly not satisfying.
- Some respondents mentioned they are not convinced a case is examined on medical grounds. The individual medical background of the patient is, at their opinion, not taken into account.
- Since SSF criteria for reimbursement are rather unclear (not clear or not enough specified, not known by the interested parties) several respondents are convinced all theoretically acceptable cases for reimbursement do not lead to final applications. The number of cases where no file is introduced is not calculable. There is a general feeling of 'under-use' of the SSF and all potential SSF cases do not result in submitted cases.
- The function of the SSF as a waiting room is not met. Drugs and treatments are kept too long within the SSF reimbursement and are not transferred to the regular health insurance system in due time. In general the procedures to have new drugs accepted into the regular health insurance system are judged as being too long and complicated.
- Acceptance of reimbursement at one time is no guarantee for later acceptance, leading to uncertainty for both patients and treating specialist doctors. There is no guarantee for the patient that the SSF will continue to finance the treatment, even in situations where the interruption of the treatment is life threatening. As such the patient is facing an uncertainty
- The name of the SSF "special solidarity fund" is not fully appropriate since the SFF only intervenes in very specific situations linked to the rareness of a disease or an indication or the innovative character of a medical technique. The fact the reimbursement or the level of the reimbursement does not depend from the patients' personal financial situation is not completely in line with the term 'solidarity'. The doctors did however not plead to have personal income of patients determining SSF reimbursement.

The main reasons why the SSF doesn't reach her objectives mentioned are:

- Too long time laps to obtain a decision from the SSF: The main reason given for this is the long duration time before a SSF file is transferred from the sickness fund to the SSF itself. Once a file is handled at SSF level, most respondents see a rather quick decision by the SSF. The bottle neck is situated at the level of the sickness funds (local sickness funds).
- Lack of knowledge and transparency on the exact definition of the SSF criteria used to judge on reimbursement. The unclearness of the interpretation by the SSF on these criteria and the unfamiliarity with the

SSF, as well within the group of medical doctors as in the group of social workers.

- The approach by the social services of the local sickness funds of SSF applications is not uniform and not transparent. The attention given to an application differs between the sickness funds and is often determined by the quality of the individual contact of the patient and his interaction with the social worker from the local sickness fund.
- Differences in treatment of SSF applications by the medical advisors of the local sickness funds creating unequal treatment of patients. The level of detailed knowledge at local sickness fund level on the SSF procedures differs substantially leading to unequal results.
- The approach by the SSF is very administrative oriented. The medical situation of the patient is rarely taken into account. The SSF decisions focus on the administrative elements of an application.
- The SFF does not start from an acceptance of the professional judgment of the treating medical doctor nor his expertise. The treating physician has too limited impact on the decision-making process of the SSF. He is not seen as an objective partner that proposes the most adequate treatment for his patient.
- The grounds, on which decisions for partial reimbursement are taken, are not known and not clear to the treating medical doctor or the patients.
- Extensive motivation of decisions is not available; there is no canvas on all decisions taken by the SSF.
- The fact a treatment has to be scientifically proven (have a scientific value and generally accepted as effective) limits the action field of the SSF. In some cases evidence based medicine is not possible since no scientific studies are or can be performed due to the rareness of the disease or the indication.

9.12.5 Alternatives for the SSF

In fact medical doctors interviewed see no real existing alternative to the SSF. However the functioning of the actual SSF is very much criticized.

In general nearly all respondents suggest a maximal integration of actual SSF reimbursement within the regular insurance system. A supplementary system as the SSF should be restrained to the ultimate minimum. Such complementary system is seen as necessary to cope with extreme unique medical situations.

Other options to respond to the needs that 'should' be met by the SSF, do not exist at this time.

Next to the SSF, different additional systems are mentioned. These are used to complement SSF reimbursement or, if no SSF reimbursement was granted to get at least maximal alternative financial support for the patient.

- Public centers for social welfare: only for patients without a minimum level of income;
- Private health insurances;
- Studies by pharmaceutical firms;
- Free samples provided by the pharmaceutical firms;
- Charitable organisations;
- The hospital (not charging the patient);
- Compassionate use of drugs;
- The cancer fund (only for small expenses).

All respondents indicated that none of these channels are a really valid and sustainable alternative to the SSF.

9.12.6 The intervention domains of the SSF

9.12.6.1 Enlargement of the SSF intervention domains

On the question "Which other elements should to your opinion be covered by the SSF?" respondents reported the following recommendations:

- Drugs not (yet) registered in Belgium should be accessible by introducing a system similar to the French system of "preliminary temporal authorization of market introduction" for new drugs. (Autorisation provisoire de mise sur le marché);
- The respondents find the criterion "rare disease and rare indication" highly limits the action field of the SSF. Exceptional medical treatments that are seen as appropriate by the treating medical doctor should be reimbursed regardless of the fact the disease or the indication is rare;
- New medication should be accepted as 'innovative medical techniques' (at this time medication is excluded) and thus also be accepted for the treatment of diseases or on indications that are not rare. If patients without a rare disease, or where strictly there is no rare indication, have a better outcome (reacting better, less side effects) when a new drug is used, they should have access to the SSF;
- Additional reimbursement of cost of treatments that are included in the regular health insurance system should exist under the SSF regulation for patients with exceptional medical cost. (ex. Physiotherapy, psychological counselling…);
- When the prescription of specific drugs is accepted and reimbursed under the SSF regulation, the additional costs of administrating the drugs should also be accepted. These costs include the use of disposables, needles, pumps, specific materials… etc. but also the specific training costs for nursing staff for administrating these specific drugs. Additional costs of hospitals for training and permanencies of nursing staff should be financed (by the SSF or by other instances). (The example of Flolan has been given);
- The criterion on "scientific value and generally accepted as effective" needs to be expanded to medical treatments showing to be effective. It is not always possible to have scientific studies for very limited patient groups (sometimes only one or a few patients) on the effects of a medical treatment. Secondly, the non administration of the drug (studies divide patient groups into 2 groups, one receiving the drug and one group receiving a placebo) would be a medical error and ethically not acceptable. When a patient has a better outcome on a drug, even if the drug is not registered for the indication, prescription and reimbursement has to be possible on a trial base. If effectiveness is proven for the individual patient, further reimbursement must be accepted.
- Proven evidence has to be individualised to proven evidence for the patient. If the individual patient reacts on a therapy, this should be sufficient for acceptance of reimbursement of the costs.
- The criterion on "experimental phase" needs to be widened. Acceptance of reimbursement of drugs should not be refused because studies are still being performed for certain indications, when the drug is prescribed for other indications. All respondents however agree financing experimental studies is not a task for the SSF.
- Drugs that are recognised as orphan drugs, but that are used for other indications than those for which the drug is accepted as an orphan drug, must be reimbursed by the SSF. The SSF has to be an additional system next to and 'on top of' the regular health insurance system. The orphan drugs (for the indications accepted by the commission on orphan drugs) are perceived as belonging to the 'regular health insurance system'

- The costs of medical treatments of a patient abroad should be expanded to 'cost of medical diagnoses'. Costs of analyses of samples have to be reimbursed, even if the patient is not transferred abroad himself.

9.12.6.2 Limitation of the SSF intervention domains

On the question "Which elements should to your opinion be excluded from the current SSF system?" all respondents see no reason to reduce the existing intervention domains of the SSF.

The exclusion of drugs or devices still in an experimental phase is accepted but limited to studies that are being performed for specific indications. It's definitely not the role of the SFF to finance experimental studies. These have to be financed by the pharmaceutical firms.

Respondents suggest to integrate as much as possible all treatments and drugs into the regular health insurance system. The SSF should be restricted to these drugs and treatment cost for which integration into the regular system is completely impossible. As such this could be regarded as a restriction of the action field of the SSF.

Off label use of drugs that are accepted under the regular health insurance system has to be kept under the regular system. There is no need to have these costs reimbursed by the SSF. They have to be reimbursed under the regular health insurance system (if this is necessary, a specific additional acceptance procedure can be foreseen in the regular system).

Five respondents explicitly suggested including the category of "chronically ill children" into the regular health insurance system. This category is not a specific target group for an additional system as the SSF

9.12.7 Experience with the SSF/knowledge of the SSF procedure

The respondents described their view on the different steps and parties involved in the SSF procedure. Most medical doctors limit their appreciation on the steps where they are implicated. The administrative elements (having the hospital invoice, provision of the original invoice from the pharmaceutical company to the hospital, the 'declaration on honor' etc.) are a responsibility of the social service of the hospital. In some cases the medical secretary of the doctors are implicated. Only in very small hospitals the medical doctor is himself confronted with these elements. In most hospitals the medical doctor has little experience on difficulties to have all the non-medical paperwork done.

- The local sickness fund is seen as the first step of the SSF procedure. One doctor said the social service of the hospital takes complete charge of the administrative steps and he only provides a prescription, a medical report and scientific articles on the treatment prescribed. Other steps are unclear to him. Contacts with the medical advisor of the local sickness fund on the specific case they introduced are possible. Nevertheless, contacts were described as very seldom;
- The internal transfer from the local sickness funds (advisory physician) to the national sickness fund level (medical director) as well as a personal contact with the medical director of the sickness fund (federal level) were mentioned;
- For most respondents it is clear a final decision on the application is taken at SSF level. The 'College of medical directors' is reported as the decision-making authority. Only one respondent said he has been contacted by a member of the college on a very specific case. (occurred only once);
- Respondents indicated that they have been informed on the follow-up of a SSF file via the local sickness fund;
- Several treating medical doctors were informed on the final SSF decision. To their opinion the information sometime was given by the patient itself. Other respondents said never having been informed on this. All respondents find it necessary to be informed on the final outcome. The fact a decision of the SSF is sent to the patient is as such understandable

but several respondents pointed out this can be problematic when a decision is negative. It can cause an unnecessary panic from the patient in a condition that is not very comfortable;

- Many respondents are aware a patient can appeal a decision by the SSF before the "labour courts" but the idea that no appeal was possible and that the only solution is to submit a new application to the SSF exist also. All respondents argument a procedure before the labour courts is totally inadequate. For patients such a procedure is heavy since in most cases they will be subject of a medical expertise ordered by the judge. Mostly the patient will also have to advance the costs of the expertise. The sickness funds are reluctant to have these procedures since at the end they would appeal a decision that was taken by their own medical director (within the SSF College of medical doctors). For nearly all respondents an appeal procedure has to be foreseen within the health system framework. Some said an appeal before a court is a total disgrace. The ruling of the courts on SSF matters should be public and accessible for doctors and patients systematically. The fact the SSF does not change its future decisions according to the courts judgments (case law) is totally unacceptable.

9.12.8 Communication and information dissemination

On the question "How do you get informed regarding evolutions/changes in the SSF regulation/procedure?" all respondents indicated that they are not systematically informed on these topics.

Not all respondents received the SSF brochure. Some respondents said the only information they have is the form they have to use for SSF files. Another said he got information from the NIHDI.

Most respondents have the social service of the hospital as administrative support and rely on the social workers for the information.

The quality of the information about the SSF is judged as difficult to access and unclear. The website where the information can be found and the documents on it are difficult to find; the website has a too complicated structure. Several respondents said medical doctors have to treat patients and do not have the time to look for information on administrative procedures.

- Most respondents indicated that the information is unclear or simply of bad quality.
- When the information was judged as "good", asking upon the SSF criteria revealed that these were totally unknown by the respective respondent.
- Several respondents expect a feedback from the SSF before a final decision is taken.

9.12.9 Knowledge and appreciation of the SSF criteria

The criteria, to be fulfilled for reimbursement of SSF applications that were reported spontaneously during the 13 interviews (without any support tool – grid) are listed below. In general spontaneous knowledge of the criteria is poor. When quoted, most respondents knew the element is used to determine reimbursement by the SSF.

- Expensive with the observation by most of the respondents that "expensive" is a very relative criterion. The level of income of the patient makes a treatment expensive or not expensive. Whether personal income should be a determinant factor for the amount or the fraction that is reimbursed by the SSF is judged very differently. Most respondents see it as irrelevant for reimbursement of medical expenses. They refer to the general health insurance system where income is not a major item for determination of the level or the amount that is being reimbursed. Several respondents made the observation that patients that are confronted with serious diseases have a lot of costs on top of the strictly medical

expenses. Most of them are not able to work and have additional expenses for mobility and housekeeping. They expect the health insurance system to fully reimburse the costs linked to the disease to the patients. Two respondents stress the opinion that reimbursement must be variable on the income status of the patient; the others don't see a need for such an approach.

- The criterion serious diseases is mentioned by five respondents, rare diseases by 4 respondents, not reimbursement within the regular health insurance system, effectiveness of the treatment, beyond the experimental phase, no other reimbursement channels, life threatening situation , added value (cost effectiveness) must be proved, scientifically proven.
- As demonstrated above, respondents could only report a limited number of criteria without the help of the criteria grid. Most respondents knew a treatment has to be expensive. One interviewee is convinced that when a treatment is very expensive, chances on reimbursement are higher. The other respondents could not agree with this at all.

The respondents were asked to formulate their appreciation of each criterion in terms of relevance (answering to a real need), clearness and formulation. On this question a grid in which all criteria were listed was shown to the respondent. One respondent said all elements were too unclear in order to give an opinion on the relevancy or the specificity of the formulation. One respondent did not complete the grid. As a consequence the overview below is based on eleven responses.

Table 21: evaluation of SSF criteria by the physicians

	Clearness			Relevant			Formulated sufficiently specific		
	Yes	Neutral	No	Yes	Neutral	No	Yes	Neutral	No
Rare indications	7		4	7	1	3	4	2	5
Rare diseases	9		2	8	0	3	6	3	2
Expensive	4	1	6	9	0	2	1	0	10
Related to a rare disease harming vital functions	8	1	2	6	2	3	6	2	3
Responding to an indication necessary on medical – social perspective	4	1	6	6	2	3	3	2	6
Scientific value and effectiveness	9	1	1	9	0	2	6	1	4
Passes the experimental stage	10	0	1	10	0	1	7	0	4
No therapeutic alternative in the regular health insurance system	11	0	0	10	0	1	8	0	3
Medical treatments abroad	10	0	1	9	1	1	8	0	3
Chronically ill children	9	0	3	6	0	5	2	3	6
Costs medical aids en treatments that are innovative medical techniques	7	1	2	9	1	1	6	1	4

NB: the occurrence of the answers is given as an indication but have no statistical signification. No inference can be done with these results due to the theoretical purposive sample, i.e. not representative in the statistical acception

9.12.9.1 Rare indication/rare disease

The criteria on rare diseases and rare indications are clear for many respondents. The criterion rare indication however is seen as not sufficiently specific in its formulation.

For both criteria the relevance is obvious. Several respondents find the rareness of the indication relevant for SSF intervention as well as for the rare diseases.

Although the criterion is seen as relevant, several respondents mentioned that reimbursement of medical costs of treatments may not be limited 'only' to rare diseases or rare indications. A patient's very specific medical condition may make the use of drugs or treatments (that are not reimbursed by the general health insurance system) appropriate. There is a general acceptance such situations have to be argued on the individual patient cases and be assessed by peers.

9.12.9.2 Expensive

The criterion expensive is not clear to several respondents while it is for others. Moreover, this criterion is not formulated sufficiently specific but it is relevant.

For almost all respondents the criterion is not totally clear since they don't know the amounts used by the SSF to determine the expensive character of a treatment, drugs or medical devices. They all would prefer to have a clear definition of the criterion.

9.12.9.3 Vital functions

For many respondents, this criterion is clear and formulated sufficiently specific. Some respondents state the criterion is too vague and has to be more specified. The relevance of the criterion is not widely accepted.

When asked to specify the criterion, the respondents mostly saw it as linked to a life threatening medical condition. Most of the respondents judged the patient has to have an acceptable level of quality of life guaranteed. It means SFF has to intervene in situations where a patient, suffering from serious medical problems can be helped to gain functional independence. A too strict interpretation of the criterion is rejected from a socio-medical point of view.

9.12.9.4 Medical – social perspective

This criterion is not clear at all. Some respondents reported that it is formulated sufficiently specific. The criterion is relevant for several respondents while others could not express an opinion on relevancy due to the unclearness of the criterion. In general from a medical point of view, not much attention is paid to this criterion.

9.12.9.5 Scientific value and effectiveness

This criterion is seen as very clear and relevant by most of the respondents. Medical doctors accept a treatment they prescribe to one of their patients has to meet standards that are scientifically valid and that the treatment (or the drug or device that is being prescribed) is accepted as appropriate and effective. The big issue here is, effectiveness and acceptability of a treatment has to be judged from a different perspective than for common diseases.

For rare diseases one cannot expect treatments to be scientifically proven the same way as for widespread diseases or medical conditions. The fact a disease or an indication is rare, in many cases makes it impossible to have the same level of scientific proof. The term 'largely accepted' is a contradictio in terminis since it concerns rare diseases and rare indications. Classic randomized studies cannot be performed since patient groups are too small or pharmaceutical firms are not interested in financing such studies since the global 'market' for the drug is judged as too small and not profitable.

In some cases it is also medically and ethically not defendable to have patients not receiving the drug since this can lead to a serious medical setback or even worse.

If a treating medical doctor is able to show a treatment, or a drug has a positive effect on the patient's medical condition, this should suffice to have effectiveness shown and reimbursement accepted by the SSF. The same counts if series of patients can be shown.

Expertise is often limited to only a few experts in Belgium. The acceptability of the decision-making 'College of medical directors' is low. Several respondents see the treating medical specialist as having a too small impact on decisions taken by the SSF. The individual medical condition of the patient is seen as not always comparable with other conditions.

9.12.9.6 Experimental stage

The criterion is clear and formulated in a sufficiently specific way to the majority of the respondents. . Many respondent judge the criterion as relevant. The remarks that were made on relevancy are that it must be accepted that studies are still ongoing for other indications and this fact may not prevent the reimbursement for other indications or very specific and special situations.

It is also obvious the SSF must accept the fact that no studies can be performed for certain indications since they are so rare randomized studies are simply not feasible.

9.12.9.7 No therapeutic alternative in the regular health insurance system

The criterion is clear to all respondents. Most of them see it as sufficiently specific and most relevant. Several respondents added the criterion must be completed with the term no 'equal' therapeutic alternative. If a patient has a better outcome using a drug or a therapy that is not part of the regular health insurance system, such treatment must be available and must be reimbursed by the SSF.

9.12.9.8 Medical treatment abroad:

This category is clear, relevant and sufficiently specific enough to nearly all respondents. The non acceptance of costs of technical exams on samples, in situations where the patient is not transported abroad, is seen as not appropriate.

9.12.9.9 Chronically ill children:

The criterion is clear to many respondents but some of them see it as formulated sufficiently specific. Several respondents see the category as a relevant criterion for reimbursement by the SSF. Most respondents don't see why this category has to be limited to children only, since children with rare diseases and chronicle conditions later become adults. One respondent said chronically ill patients are not really a target group for a special solidarity fund. They must preferably be integrated in a clinical path. Several respondents think these costs can easily be integrated into the regular health insurance and should not remain a part of SSF reimbursement.

9.12.9.10 Innovative medical techniques:

The category is clear to many of the respondents. The criterion is judged as highly relevant but the formulation of the criterions is slightly doubted. One respondent said the exclusion of drugs as innovative medical techniques is questionable. In fact the statement means the respondent sees no reason why reimbursement of drugs should be linked to the rareness of the disease or the indication. As we mentioned above, this statement was also made for the criteria rare disease and rare indication.

Also the term innovative may not be equal to new. Prescription of existing devices or treatments (medical techniques) outside the criteria as foreseen in the regular health insurance system should be allowed and reimbursed for very specific medical conditions.

9.12.10 The internal procedure within the hospital

9.12.10.1 The initiator and final responsible for SSF applications

- The medical doctor is reported as the principal initiator of SSF files as well as the social service as additional/complementary initiator. More, the social service of the hospital drew the attention on the existence of the SSF. The pharmacy department, the invoice department from the hospital and a pharmaceutical company were also mentioned as an initiator. In some cases patient organizations can influence indirectly by attending the medical doctor on specific costs he is not completely aware of. Most medical doctors treating patients with rare diseases have contacts with patient organizations.
- The final decision to submit a file to the SSF is definitely a medical one. All respondents said they take the ultimate decision to introduce an application for reimbursement at the local sickness fund (SSF procedure). Several respondents explicitly mentioned they start the treatment at the same time and do not wait for a decision by the SSF while one respondent said he never starts the treatment before the patient obtains a decision because the costs are too high to be supported by the patient.
- Most medical doctors give information on the possibility of acceptance of the application by the SSF. No respondent gives full assurance the application will be accepted. They mostly tell the patient they will try but that decisions of the SSF are difficult to predict. In some cases the respondents give a probability based on earlier experiences but always add there is no guarantee at all the SSF will grant reimbursement. Several respondents said they had contradictory ruling by the SSF in the past. Most respondents inform the patient he will receive a bill from the hospital for sometime very high amounts. They warn the patient that they do not have to pay instantly and that the bill is needed to be able to introduce an application at the SSF. Most hospitals grant a delay of payment to the patients. Even if the SSF refuses the petition for reimbursement, the hospitals try to help patients to overcome that situation (payment plan). All respondents say this is a very annoying situation where the patient is put in a very stressful situation.
- If a patient does not want to take the financial risk, even if that risk is theoretical, the medical treatment has to be postponed. This is seen as not acceptable at all. Three respondents mentioned they were confronted with such situations.
- It is not always possible to inform the patient on the financial consequences of a medical treatment. The patient can be in a very critical condition where dialoguing is not possible or not convenient. This poses ethical dilemmas for the treating doctor.

9.12.10.2 More or less involvement in the procedural steps

All but one respondent want to have more involvement in the decision-making process. They want to be seen as an objective partner and even they deem it necessary to be contacted by the SSF if a negative decision is envisaged. Some respondents said they want to be able to meet with the College of medical directors to argument the case. Most doctors see this as not feasible since they do not have the time to do this. They must treat patients, not act as a counselor.

It is clear the decision-making process of the SSF is very much questioned by the treating medical doctors. They see no added value from the advice of the advisory doctor at local sickness fund level. The medical expertise needed to judge on such complicated cases as these introduced for reimbursement by the SSF, just is not present at this level. This is very well understandable since one cannot be an expert for all diseases.

The respondents have no contact with the medical directors of the sickness funds at federal level. They also have no contact at all with the medical staff at SSF level. Nearly all respondents are convinced the needed medical expertise to judge on very specific patient files, is simply not present at the decision table.

All respondents agree to have a review of the treatment, the drugs or the devices they prescribe but want this review to be processed by making use of other means than the actual SSF decision-making council. They propose to have experts (national or international) involved or a second opinion process. The expertise on the diseases or the indications is often very limited in Belgium. They all consider themselves as experts and have great difficulties that their medical judgment is questioned by non-peers.

For routine SSF files, this may not cause major problems, but for very complicated cases, a system of peer review and peer decision has to be put in place.

They however do not want to have a similar procedure as the procedure on orphan drugs. This procedure is judged as too heavy and too restricted. The fact every year a petition for renewal has to be entered is seen as unnecessary complication of the procedure. The paperwork for the orphan drugs is seen as even more heavy as for the SSF applications.

Several respondents referred to the French system where new drugs, which are not generally accepted in the health insurance system (yet), can be prescribed to certain groups of patients, or for other indications without heavy procedures. Decisions are taken very quickly and they can make use of the ATU procedure to have access to drugs that are not registered yet in France.

The one respondent that did not want further involvement argued his statement saying for him the SSF applications in fact are routine files where acceptance is evident and needs no further argumentation. There is enough paperwork already.

Respondents want to decrease their involvement in SSF files, and formulated the following suggestions/remarks in order to increase efficiency and simplify the current procedure:

- The administrative burden is judged to be high by many respondents. Most responders understand there is a minimum of paperwork that has to be done for every application for specific reimbursement of medical costs, but it has to be limited to the strict minimum. Medical doctors are overwhelmed with paperwork when their first task is to treat patients, not filling out all sorts of formularies;
- Duplication of scientific articles and literature on the SSF cases is seen as an unnecessary waste of time. The SSF can easily keep track of all elements provided on similar previous cases and as such avoid unnecessary duplication. Respondents do not see the need to have these provided on each separate application;
- The fact prices have to be given and costs have to be calculated each time is also seen as unnecessary complication. It creates an unproductive administrative burden. Prices can be provided by the NIHDI and cost calculation could be performed by the SSF administrative services. It should be sufficient if the prescribing doctor gives the dose that has to be administrated to the patient. Documents have to be regularly re-entered to the sickness funds when lost. Several respondents mentioned this fact. In Belgium no financial compensation at all is given to medical doctors for all the paperwork on patients' files. In other countries as Luxembourg it is.
- The administrative step of the local sickness funds is completely unnecessary and gives no added value for the SSF process at all. Most respondents prefer to have SSF files entered at the SSF services directly. It would be much easier to have one single point of contact. Several respondents judge the intermediate step of the local sickness fund as completely unnecessary. It only consumes more time and resources and

adds more unnecessary paperwork to the process. It only makes the time for a final decision longer. The only added value of the local sickness fund is the accessibility for the patient but since knowledge at local sickness fund level is low and the real initiator is the treating physician, there is no real need for this.

- Entering applications directly to the SSF would avoid duplication of work. Now an application is screened a first time at the local sickness fund level, a second time at the sickness fund at national level and a third time at SSF level (administrative services). There is no added value from the advice of the medical advisor of the local sickness fund. Respondents see no need to have the files transit at federal sickness fund level. Shortening the administrative pathway can shorten the period towards a decision.
- The appeal procedure for SFF decisions is not appropriate. There should be an internal appeal procedure avoiding having to refer to the courts of law. Such an external procedure should be limited to very exceptional situations (last resort).

9.13 RESULTS FOR THE INTERVIEWS WITH PHARMA.BE AND REPRESENTATIVES OF FOUR PHARMACEUTICAL COMPANIES

9.13.1 General remarks Pharma.be

The Belgian pharmaceutical sector sees a major problem in the fact that Belgium does not have a specific system for "early access" to new drugs. For medication that already has EMEA registration but where there is no reimbursement decision at Belgian level, the only possible solution for having the costs reimbursed is to introduce an individual application, at individual patient level, at the SSF.

In other countries as France an "early access system" exists as well at individual patient level, as at patient group level (ATU - Authorisation temporaire d'utilisation). A pharmaceutical company can introduce such a demand for early access in which case the use of the new drug can be authorised for specific patient groups and/or indications linked to a therapeutical protocol. In such a case, the pharmaceutical company has to engage itself to introduce a demand for reimbursement at a later stage. The use of the drug and reimbursement of costs however is already temporarily regulated. The pharmaceutical industry proposes to have such an early access system introduced in Belgium too. They do not think the SSF is the adequate instrument to provide such an early access since the SSF is limited to only case by case decisions.

Most pharmaceutical companies understand that when a drug has not yet obtained the EMEA marketing authorisation, and the drug is needed for the treatment of an individual patient, the costs cannot be charged to the national health system. But if market authorisation has been obtained, they find it's the responsibility of the public health care insurance system to cover the costs. They also ask to shorten the time that is needed for acceptance of new drugs (after EMEA registration) into the Belgian health care insurance system.

Compassionate use and medical need programs:

Compassionate use and medical need programs are not seen as a structural solution for these situations. Both result in the fact that only the pharmaceutical company bears the cost of these drugs. For some smaller Belgian companies this is not obvious. One company mentioned compassionate use counts for about 1/12th of their gross sales in Belgium. The difference between compassionate use and medical need program for the company is, the compassionate use is completely at charge of the Belgian company as for the medical need program the costs are at charge of the "mother company".

- Compassionate use occurs when a drug has not obtained the EMEA market authorisation yet (for the drug itself or for the very specific indication for which it will be used for the treatment of the patient). The pharmaceutical companies say that for rare indications it is not obvious to

ask for EMEA market authorisation. The cost for the studies to be performed is mostly too high in perspective of the number of cases concerned. In some cases scientific studies just cannot be performed since the patient groups are too small.

- Compassionate use is certainly no structural solution for off label use of medication. Strictly compassionate use is limited to a one time use of the drug and leads to inequality among patients since not all of them that are in the same situation of a medical need, will have access to the drug. The pharmaceutical companies ask for the introduction in Belgium of a structural system that regulates off label use. They refer to the systems in the surrounding countries where off label use is regulated and a procedure for reimbursement exists.
- The medical need programs concern the use of a drug that already obtained EMEA market authorisation but has not been accepted for reimbursement by the compulsory health care insurance system. The acceptance in Belgium requires a decision on acceptance of the drug and a second one on the price of the drug.

9.13.2 Suggestions of the pharmaceutical companies

- Risk sharing system:

  This system exists in the UK and in Italy. It means the public health care insurance system will only fund the costs of the medication if the patient reacts favourably to the treatment. At first there is no reimbursement, later if the reaction on the treatment is positive, the drug will be reimbursed. The difficulty in such a risk sharing system is that it is not always clear what 'reacting on the treatment' implies. Criteria for determining if the patient reacts positive to the treatment are difficult to define. Such a system has to be transparent but may not put a too high administrative burden on the prescribing doctor. This seems to be the case in the UK with the consequence the system is not used as it should be.
- Stopping rules:

  Such a system allows the prescribing doctor to use the drug and see if the patient reacts positive to the treatment. At the first stage the drug will be reimbursed. If the patient reacts positive, the doctor has to deliver a declaration the patient does and the treatment can be continued. If not the treatment and the reimbursement will be terminated.

9.13.3 The view of the pharmaceutical companies on the SSF

The SSF is seen as a system that provides solutions to patients for high medical expenses that are not covered by the compulsory health care insurance system. As such the existence of a kind of safety net is judged as positive. The pharmaceutical sector however formulates different remarks as to the functioning of the SSF.

Transparency of the criteria used by the SSF is poor. Even doctors and certainly patients do not understand the criteria nor the way the SSF applies these criteria on individual cases. SSF decisions are case by case decisions without any guarantee on later acceptance. It would be more adequate if decisions of the SSF would be public (anonymous) and linked to the pathology and the indications. In such a case more certainty on acceptance would be available.The administrative burden for the prescribing medical doctor is very high and leads to cases where no application is introduced although theoretically the SSF could intervene.

Decisions at the SSF are taken by the College of medical directors. Since it mostly concerns rare diseases or rare indications, one cannot expect them to have all sufficient knowledge that is needed for an adequate judgment of each individual case. The advice of the Drug Reimbursement Commission (DRC/CTG/CRM) is no solution since the members of this committee do not have the expertise on innovative new drugs or on rare indications either.

The time between the moment where the medication is needed for the treatment of the patient and the moment where a decision on reimbursement is taken, is judged as unacceptably long. The internal procedures of the SSF as well as the preliminary steps at sickness fund level are not clear to the respondents.

The pharmaceutical companies are not involved in the SSF procedures, even if they have to deliver expensive drugs at the start of a patients' medical treatment. Indeed, when asked for, the pharmaceutical company has to deliver the drug to the patient via the hospital at the moment the drug is needed for the treatment of the patient. In most cases the need for the drug is urgent and the drugs have a high cost. Ethically the company cannot refuse to provide the drug, even if at this stage the company has no guarantee at all the product will be paid for.

In most cases the pharmaceutical company will make an invoice to the hospital for the drugs that have been provided. In reality the invoice will not be paid for as long as there is no decision from the SSF and payment is effectively received by the hospital. If the SSF only refunds a percentage (mostly 60%) of the price of the medication, the hospital will only pay that part of the invoice to the pharmaceutical company. The rest of the amount stays 'open'. The laps of time between the provision of the drug and the partial payment by the hospital in general exceeds the period of one year. It can take several years before the additional 40% is paid. In most of the cases the 40 % will be limited to the price that finally is accepted by the SSF. The company does not know when the additional reimbursement is executed.

The pharmaceutical company has no knowledge or control at all on the introduction of an SSF application since the application is initiated by the patient (via the medical doctor that prescribed the drug). No information is given on the fact if an application is submitted to the SSF or on the progress of the decision-making process. The pharmaceutical company does not know if the prescription of the drug meets the criteria as applicable for SSF files. They can not verify if the patient's medical condition meets the criteria of the SSF and if or when a decision by the SSF has been taken. The outcome is not known to them. They fully depend on the information that is given by the hospital. Contacts with the SSF itself are not allowed. For the pharmaceutical companies it is clear the actual system leads to many discussions between the company, the hospital and the patient. Uncertainty for all is very important and not acceptable.

The administrative follow-up of the invoices for the medication to the hospitals is judged as very time consuming. It can take several years. In some cases the total amount of these invoices that are waiting for payment can be very important. One company mentioned the amount of €4,5 M for one type of drugs over a period of two years. If the SSF takes a negative decision and the reimbursement is refused, the pharmaceutical companies say they mostly will not be paid. They find it unreasonable that they have to bear the risk in such cases; even if they understand these (mostly very high) costs cannot be charged of the patient.

The SSF procedure normally foresees the reimbursement will be done to the patient. If the patient agrees, the payment will be executed directly to the hospital (the hospital pharmacy that delivered the drug to the patient). As mentioned, the pharmaceutical company is not informed of the status of an SSF application and gets no notification of a decision taken by the SSF. They also don not know if the patient agreed to have a direct payment to the hospital. The pharmaceutical companies ask to have a more transparent procedure where, if they delivered the drugs and have not been paid for it, the SSF would keep them informed on the decision and the payment procedure. It must be possible to organize such notification in full respect of the patients' privacy by making it completely anonymous.

9.14 INTERNATIONAL COMPARISON: FRANCE

9.14.1 Sources

- Telephone interview with Mrs. Marie-Claude Hittinger, Rare diseases Expert of the Haute Autorité de Santé
- Telephone interview with Mrs. Isabelle Cheiney, Direction de la Sécurité Sociale of the French Ministry of Health
- Telephone interview with Mrs. Anna-Marie Bouchard, Comité Economique des produits de Santé (CEPS)
- Circulaire Assurance Maladie – Caisse nationale regarding the conditions for reimbursements of costs, related to rare diseases. .
- Website:
  - Comité Economique des produits de Santé (CEPS) : http://www.sante.gouv.fr/ceps
  - Assurance Maladie : http://www.ameli.fr/

9.14.2 Short introduction to the French healthcare system

9.14.2.1 Who is covered?

In France, one should distinguish between:

Basic, public health care insurance, providing a standard benefits package for all residents (assurance maladie obligatoire). There is a universal, basic health care insurance providing a standard benefits package for all residents, through large occupation-based funds. One can distinguish between different public insurance schemes:

- The General National Health Insurance Scheme (le régime général de la Sécurité sociale) covers about 83% of French workers, as well as their families, and is the most important one.

Other occupational specific schemes cover the remainder:

- La Mutualité sociale agricole (MSA) covers agricultural workers and their families - about 9% of the population.
- La Caisse nationale d'assurance maladie des professions indépendants (CANAM) covers artists, entrepreneurs and other self-employed – about 6 of the population.

Following the introduction of Couverture Maladie Universelle (CMU) in 2000, the state finances coverage for those residents that are not eligible for coverage by the public health insurance scheme (0.4% of the population). The state also finances health services for illegal residents (L'Aide Médicale d'Etat; AME).

Complementary private health insurance: in order to cover the difference between what the state pays and the cost of treatment (complément), most French residents purchase a complementary health insurance policy to cover the difference. This is called a police complémentaire or mutuelle and is provided by about 80 insurance companies, 20 welfare institutions (institutions de prévoyance, managed by both employers and syndicates) and 6500 mutual societies (mutualités). Often, complementary health insurance is occupation-based. Complementary private health insurance covers over 92% of the population. In 2005 out-of-pocket payments and private health insurance accounted for 7.4% and 12.8% of total health expenditure respectively.[45]

9.14.2.2 What is covered?

The public health insurance scheme covers hospital care, ambulatory care, prescription drugs, as well as minimal coverage of outpatient eye and dental care.

Cost-sharing: The principle of cost-sharing, or ticket modérateur, is extensively applied to public-financed health services in France. There are three forms of cost-sharing: co-insurance, co-payments and extra billing.

Co-insurance rates are applied to all health services and drugs listed in the publicly-financed benefits package and vary depending on:

- type of care: hospital care (20% plus a daily co-payment of €16), GP visits (30%), dental care (30%);
- type of patient: e.g. patients suffering from chronic diseases and low income patients are exempt from cost sharing;
- effectiveness of the prescription drug: 0% for highly effective drugs, 35%, 65% or 100% for drugs with a limited therapeutic value;
- level of compliance with the current gate keeping system (médecin référent): co-insurance regulation tends to favour visits to people's 'médecin référent' (30% co-insurance rate) to visits to other GPs (50%).

As mentioned, treatment costs are split between public health insurance (what the state pays) and the patient (co-payment). However, payments by patients can be reimbursed by complementary private health insurance, up to 100%. From 2008, some smaller co-payments ceased being eligible for reimbursement up to an annual ceiling of €50.

Reimbursement by the publicly financed health insurance scheme is based on a reference price (tarif de convention). Although a patient may be treated by a practitioner classed as 'conventioné', he can ask more than the tariff de convention provided it is "reasonable and tactful" to do so. For example, a specialist who has extra qualifications or experience in other countries could charge extra for its services. This supplementary charge is called a 'depassement' or **extra billing**. The difference between the extra billed amount and the reference price is not covered by public health insurance, nor complementary private health insurance and is fully charged to the patient.

Safety nets: France introduced some safety nets for people with invalidity or with work injury, people with specific chronic illnesses and low income patients. These groups of citizens are exempt from co-insurance regulations and are reimbursed at 100% of the "tarif de convention" if one of the following applies:

- A patient suffers from one or more of the 30 chronic or acute medical conditions that are listed, including insulin-dependent diabetes, cancer and heart disease;
- A patient requires long-term care or care for a long-standing condition (called an ALD–affectation de longue durée);
- A patient is hospitalised beyond 30 days. From the 31st day hospital stays are 100% reimbursable;
- A patient's surgery costs are above €91.

Children and low income residents are exempt from paying non-reimbursable co-payments.

Complementary private health insurance (police complémentaire), by most of the patients obtained through their employer, covers patients' statutory cost-sharing, meaning the share of health care costs that is not reimbursed by the public health insurance scheme. However, this is only the case for health services and prescription drugs that are part of the publicly-financed benefit package.

Low income residents (for example, €10,768 for a couple) that cannot afford a police complémentaire could rely on free complementary state-funded healthcare, called CMU complémentaire. They cannot be extra billed by doctors.

Financing of the health system: The public health insurance scheme is financed by employer and employee payroll taxes (43%); a national income tax (contribution sociale generalisée); created in 1990 to broaden the revenue base for social security; revenue from taxes levied on tobacco and alcohol (8%); state subsidies (2%); and transfers from other branches of social security (8%).

9.14.3 The organization of healthcare provision

In comparison to the situation in the Netherlands, **the Government** has an important and steering role in the management of healthcare system in France. Sometimes, it intervenes directly in the production and financing van healthcare services. Moreover, it strongly supervises the interactions between healthcare providers, patients and health insurers.

Every year the **French parliament** sets a maximum limit for the rate of expenditure growth in the public health insurance scheme for the following year.

Health insurers have a public character and are managed by a board of representatives, with equal representation from employers and employees. Public health insurance funds are statutory entities and their membership is based on occupation. Hence, there is no competition between them. There is no system of risk adjustment among them like it is the case in the Netherlands, even though there is inadvertent risk selection based on occupation.

It should be noted that there is limited competition among mutual associations providing complementary private health insurance.

Physicians are self-employed and paid on a fee-for-service basis. The cost per visit is slightly higher for specialists (€23) than for GPs (€22) and is based on negotiation between the government, the public insurance scheme and the medical unions. Depending on the total duration of their medical studies, physicians may charge above this level.

The 2004 reform of health financing in France introduced a voluntary gate keeping system for adults, known as "médecin traitant". However voluntary, there are strong financial incentives favouring visits to the referent GP.

Two-thirds of **hospital** beds are in government-owned or not-for profit hospitals. The remainder is private for-profit. All university hospitals are public. Hospital physicians in public or not-for profit facilities are salaried.

Public hospitals are currently financed by a general budget that also includes all medicines that are used within the hospital. Some expensive and innovative medicines are financed by special financial resources that are accorded by the Minister of Health. Private hospitals are financed on the basis of a day-price, covering costs of stay and nursing, and of a flat-premium covering expenditure for medicines. Compared to public hospitals, honoraries are charged separately. From 2008, all hospitals and clinics will be reimbursed via the DRG-like prospective payment system, like it is the case in the Netherlands. Public and not-for-profit hospitals benefit from additional non activity-based grants to compensate them for research and teaching and other additional services.

As mentioned, interactions between the social security system and the healthcare providers are based on conventions, setting out the framework for compensations and reimbursements. Conventions are the product of negotiations between the representative employees' organisations and representatives of the important public health insurance schemes.

Based on these conventions, healthcare providers charge their delivered services in function of two elements: the classification (nomenclature) of groups of services based on their nature, and the negotiated price for these different service groups. The Nomenclature lists also all medicines that are reimbursed by public health insurance to the patient.

Updates of the classification system require a formal decision of the French government and the preliminary advice of a Commission, composed of healthcare providers representatives and health insurers (Comité économique des produits de Santé).

9.14.4 List of Chronic Diseases (Affection de Longue Durée - ALD 30)

Accident vasculaire cérébral invalidant

Aplasie médullaire et autres cytopénies chroniques

Artériopathies chroniques avec manifestations ischémiques

Bilharzioze compliquée

Insuffisance cardiaque grave, troubles du rythme graves, cardiopathies valvulaires graves, cardiopathies congénitales graves

Maladies chroniques actives du foie et cirrhoses

Déficit immunitaire primitif grave nécessitant un traitement prolongé, infection par le Virus de l'Immuno-déficience Humaine (VIH)

Diabète de type 1 et diabète de type 2

Formes graves des affections neurologiques et musculaires (dont myopathie), épilepsie grave

Hémoglobinopathies, hémolyses, chroniques constitutionnelles et acquises sévères

Hémophilies et affections constitutionnelles de l'hémostase graves

Hypertension artérielle sévère

Infarctus coronaire

Insuffisance respiratoire chronique grave

Maladie d'Alzheimer et autres démences

Maladie de Parkinson

Maladies métaboliques héréditaires nécessitant un traitement prolongé spécialisé

Mucoviscidose

Néphropathie chronique grave et syndrome néphrotique primitif

Paraplégie

Périarthrite noueuse, lupus érythémateux aigu disséminé, sclérodermie généralisée évolutive

Polyarthrite rhumatoïde évolutive grave

Affections psychiatriques de longue durée

Rectocolite hémorragique et maladie de Crohn évolutives

Sclérose en plaques

Scoliose structurale évolutive (dont l'angle est égal ou supérieur à 25 degrés) jusqu'à maturation rachidienne

Spondylarthrite ankylosante grave

Suites de transplantation d'organe

Tuberculose active, lèpre

Tumeur maligne, affection maligne du tissu lymphatique ou hématopoïétique.

9.15 INTERNATIONAL COMPARISON: THE NETHERLANDS

9.15.1 Sources

9.15.1.1 Interviews

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- 2 telephone interviews with Mrs. Anneke Prenger, Senior Advisor Pharmaceutics, Dutch Federation Health Insurers (Zorgverzekeraars Nederland), September 2009
- 1 telephone interview with Mrs. Jolande Huizer, Steering Committee Orphan Drugs (Stuurgroep voor Weesgeneesmiddelen, September 2009

9.15.1.2 Websites

- Dutch Federation of Health Insurers/Zorgverzekeraars Nederland: http://www.zn.nl/OverZN/index.asp
- Files with respect to the reforms of the health system and health Insurance system, NRC Handelsblad: http://www.nrc.nl/dossiers/zorgstelsel/de_zorg_instellingen/
- Dutch Ministry of Health: http://www.minvws.nl/
- Dutch Steering Committee Orphan Drugs (Stuurgroep voor Weesgeneesmiddelen): http://www.weesgeneesmiddelen.nl/
- Dutch Healthcare Insurance Board / College van Zorgverzekeraars: www.cvz.nl
- Dutch Federation of Health Insurance Providers / Zorgverzekering Nederland: http://www.zn.nl/OverZN/index.asp
- Dutch Organisation for Health research and Healthcare innovation: www.zonmw.nl
- Rare Diseases fund: www.zzf.nl
- European Observatory on Health Systems and Policies, http://www.euro.who.int/observatory

9.15.1.3 Publications

- The Commonwealth Fund, Descriptions of healthcare systems: Denmark, France, Germany, The Netherlands, Sweden and the United Kingdom, 2008
- The Commonwealth Fund, Descriptions of healthcare systems: Denmark, France, Germany, The Netherlands, Sweden and the United Kingdom, 2008
- Steering Committee Orphan Drugs, Beschikbaarheid van Geneesmiddelen voor Zeldzame Ziekten, www.weesgeneesmiddelen.nl,2009

9.15.2 An introduction to the Dutch healthcare system

9.15.2.1 Who and what is covered?

The Dutch health insurance system can be divided in three components.

- Statutory Exceptional Medical Expenses Act scheme (Algemene Wet Bijzondere Ziektekosten, AWBZ), covering a wide range of chronic and mental healthcare services with an exceptionally high cost. All Dutch residents are covered by the Act.
- Statutory health insurance, providing a standard benefits package, based on the Health Insurance Act (Zorgverzekeringswet, 2006).
- Complementary health insurance, covering less vital healthcare services that are not covered by the standard benefits package (dental care for people above 16, alternative ways of treatment, etc.)

Since January 1, 2006, all residents or those paying income tax in the Netherlands are required to purchase health insurance coverage. If not, they risk penalisation. Insurers are legally required to provide a standard benefits package covering medical care; hospitalization; dental care (up to the age of 18); medical aids; medicines; maternity care, ambulance and patient transport services; paramedical care.

In addition to the statutory standard benefits package, most citizens also have complementary private health insurance for specific services that are not covered by the standard benefits package - for example dental care (>age 22) or alternative treatments. In opposite to the statutory health insurance, insurers are not required to accept applications for private health insurance and could refuse potential clients. As most of the population purchase a mixture of complementary and supplementary private health insurance from the same health insurers who provide statutory coverage, the government is concerned about the potential for risk selection (premiums and products of voluntary coverage are not regulated).

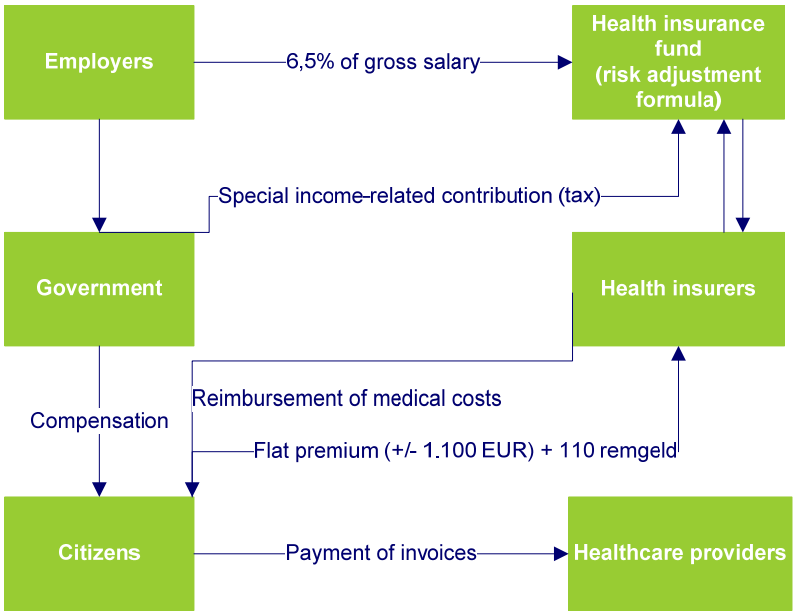
9.15.2.2 Cost-sharing

Insured people with the same policy pay a same flat-rate premium to their private health insurer. Before 2007, an insured was eligible for a refund of €255 if they incurred no health care costs. If they incurred less costs than €255, they received the difference at the end of the year. This system, called 'no claims bonus' system, was abolished in 2007 and has been replaced by a system of deductibles. Every insured aged 18 and over must pay the first €150 of any health care cost in a given year. Children are exempt from cost-sharing. The government introduced the use of 'health care allowances' for low income citizens if the average flat-rate premium is higher than 5% of their household income.

9.15.2.3 Financing of the health system

The statutory health insurance system is financed by premiums (almost 50%), paid by the insured to his health insurer, and by income-related contributions. Income-related contributions are collected by the Government and distributed among insurers following a risk-adjusted capitation formula. Because the complementary health insurance system fully drives on market mechanisms (no premium regulation), the risk-adjusted capitation formula is not applicable to complementary health insurance.

Figure 21: Financing of the health insurance system (Statutory health insurance system)

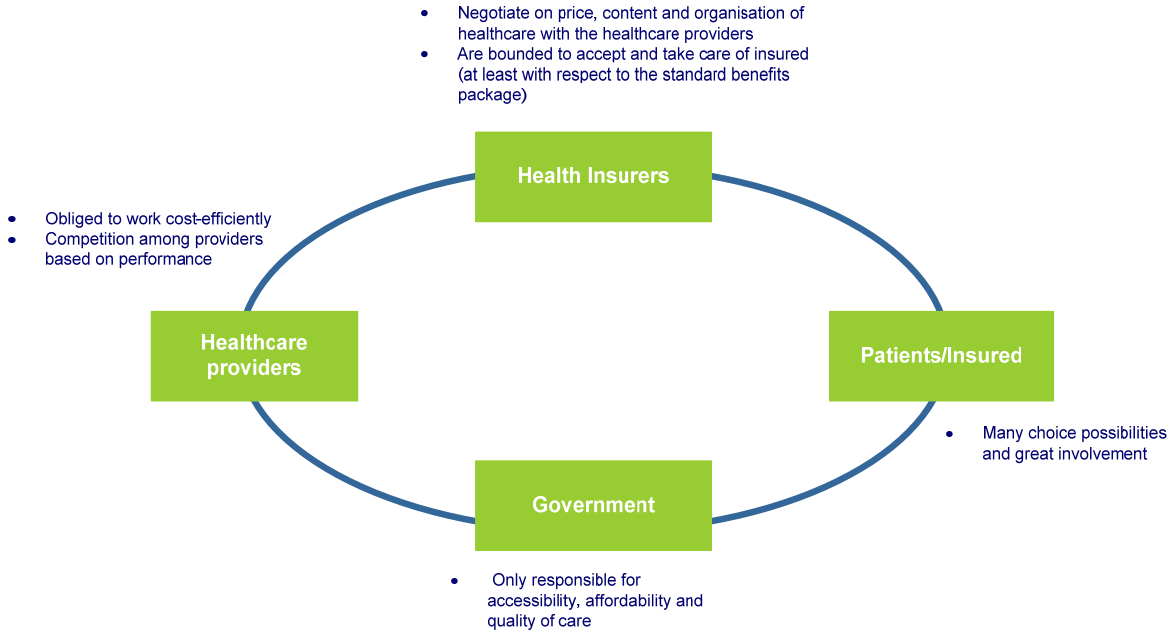


Source: Christelijke Mutualiteit (CM), Gezondheidssystemen in Europa, Ziekteverzekering en gezondheidszorgen in Nederland, maart 2008

In 2005, public sources of finance accounted for 65,7 % of total health expenditure, a percentage that has risen to 78% in 2006. In 2005, private health insurance accounted for 20,1% of total health expenditure, a proportion that had fallen to about 7% in 2006.

9.15.2.4 *The organization of healthcare provision:*

Figure 22: Involved parties in the Dutch healthcare system



Source: own analysis

Health insurers

The Dutch health insurers, about 30 are active in the Netherlands, are private actors that are allowed to make profits. However, registration with the Supervisory Board for Health Insurance (CTZ) is required to qualify for payment from the risk equalization fund. Indeed, with respect to the standard benefits package, insurers are compensated by the Government for accepting high-risk patients through a solidarity mechanism (Centrale Kas / Risicovereveningsfonds – Central Fund / Risk compensation Fund).

With respect to the standard benefits package (provided by the statutory health insurance), all health insurance providers (zorgverzekeraars) are bounded by acceptance obligations (aanvaardingsplicht), which means that they cannot refuse patients or patient groups as client. In exchange for the acceptance obligations, the Dutch government compensates the risks that health insurers face of having a higher number of risk patient groups in their clients base (patients with a relatively high probability of relying on sickness funds). This risk compensation system, or risicovereveningssysteem in Dutch, is applied on both ex-ante (Hogekostenverevening – HKV) and ex-post estimates (hogekostencompensatie – HKC). The definition and categorization of risk patient groups is not explicitly limited to rare diseases, but is broadly defined to all costly chronic diseases. Health insurance providers appear to identify about 3-5% of their clients as high potential risks. Hence, health insurance providers reimburse the costs for drugs and treatment to older patients or patients with rare and chronic disease. The Dutch Government compensates health insurers for the risks they are taking with accepting specific patient groups. For patients that suffer from rare diseases, the compensation by the Government to insurance providers can amount to 90% of the total costs.

Healthcare providers

Physicians are directly or indirectly contracted by private health insurers, based on bilateral negotiations. GPs receive a capitation payment for each patient on their practice list and a fee per consultation. Most specialists are hospital-based. Two-thirds of hospital-based specialists are self-employed and are paid on capped fee-for-service basis. The rest is salaried. Experiments with pay-for-performance are just a matter of time.

Most Dutch hospitals are private non-profit organizations. Hospitals are increasingly encouraged to obtain capital via the private market. From 2000, payments to hospitals were rated according to performance a number of accessibility indicators, a measure designed to reduce waiting lists. A new system of payment for specific products, called the Diagnosis Treatment Combination (DTC), is currently being implemented. This new system introduces a system of hospital output prices that are based on the production process rather than on budgets and financing rules. The system based on Diagnosis and Treatment Combinations (DTC) is expected to solve existing problems with insufficient production incentives and a lack of efficiency within the hospital.

Government

With the 2004 reform of the healthcare system, the Dutch government introduced a so-called 'regulated competition' among health insurers, based on the idea that competition among health insurers (but also among healthcare providers) would decrease healthcare expenditure and result in a more cost-efficient healthcare provision.

As the healthcare providers and insurers are operating in a system where they have to negotiate with each other on price and content of medical care, the role of the Government has evolved towards supervision and setting frameworks.

9.16 INTERNATIONAL COMPARISON: SPAIN

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9.16.1.1 Publications

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- Plan de atención a personas afectadas por enfermedades raras 2008-2012, Consejería de Salud, Junta de Andalucía.
- Acceso a medicamentos en situaciones especiales, Javier Bautista Paloma; VI Jornadas de ensayos clínicos Andalucia 2009, Málaga 25-26 junio.
- Acuerdo del Pleno del Senado por el que se aprueba el Informe de la Ponencia de estudio encargada de analizar la especial situación de los pacientes con enfermedades raras y, especialmente, las medidas sanitarias, educativas y sociales que contribuyan a un adecuado tratamiento de los enfermos y de sus condiciones de vida, aprobado por la Comisión conjunta de la Comisión de Sanidad y Consumo y de la Comisión de Trabajo y Asuntos Sociales en su reunión del día 18 de diciembre de 2006 (543/000016 BOCG, Senado, serie I 23 de febrero 2007 Num. 659.

9.16.1.2 Interviews

- Telephone interview Dr. Javier Bautista Paloma, Chief Farmacian Hospital Universitario Virgen del Rocio, Sevilla.
- Telephone interview Sra Estrella Guerrero Solana, FEDER (Federación Española de Enfermedades Raras - Spanish Federation for Rare Diseases) Avda. San Francisco Javier, 9 Pl. 10 M.24 (41018) Sevilla and mail from D. Moisés Abascal, vicepresidente FEDER.
- Telephone interview Sr.Ester Tallon, Agencia Española de Medicamentos y Productos Sanitarios.
- Telephone interview Sra Sandra Flores, Agencia de Evaluación y Technologia Sanitaria.
- Telephone interview and mail Ministerio de Sanidad y Consumo, Madrid, Mª Dolores Vaquero García; Subdirectora general de calidad de Medicamentos y productos sanitarios Dirección General de Farmacia.

9.16.1.3 Websites:

- www.enfermedades-raras.org FEDER
- www.agemed.es Agencia Española de Medicamentos y Productos Sanitarios
- www.msc.es Ministerio de Sanidad y Política social

9.16.2 Introduction to the Spanish healthcare system

The Spanish health system is organized at different levels. Since the transition to democracy one of the main elements of the country's transformation has been a profound political decentralization of the state structures. The organisation of the Spanish state is made up of the central state and 17 autonomous communities (Comunidades Autónomas). The autonomous communities are highly decentralized regions, each of them with their own regional government and parliament. They have gained competences on important issues and have become crucial in the organisation of the public healthcare system.

The Spanish Constitution of 1978 established the right of all citizens to health protection and set out a new regionally based organizational framework. The constitution recognizes the right of all citizens to adequate public health services. It introduced the territorial division of powers between the central state and the regions in the fields of public health and health care.

This political structure has great impact on the organization of the Spanish public health system. Legislation on public health and health care provision, since is as well at national as at regional level.

The basic law regulating the public health system is the General Health Care Act of 1986.[46] It outlines the main principles for the Spanish National Health System (NHS).It also introduced a formal transition from a system of social security to an NHS model, with a progressive transition from payroll contributions to general taxation as the main source of financing.

The general principles of the National Health System are:

- universal coverage with free access to health care for almost all inhabitants
- public financing (mainly through general taxation)
- integration of the different former national health service networks under the National Health System structure (INSALUD)
- political devolution to the autonomous communities and region-based organization of health services into health areas and basic health zones

Today the 17 autonomous communities have considerable legislative power and autonomy with regard to health care policy and most social services. The devolution of powers to the regional level (the autonomous communities) has been realized over a rather long period. The transition from the national level (former INSALUD - Central National Health Institute) to the regional level (regional health services depending from the regional governments) took more than 10 years. It was implemented gradually per autonomous region. Cataluña was the first autonomous region to have the public health competences transferred to in 1981. The last transfers took place in 2002 when the decentralization process was finished.

The Autonomous Communities are responsible for the delivery of their own health services. Through their Regional Health services they are the administration level in charge of health management and control. They have the authority to decide how to organize or provide health services. Every citizen adheres to the Regional health service of the region he lives in. It's the regional health service that is responsible for the organization of the primary care as well as the hospital care and emergency care.

At central level, the Spanish Ministry of Health and Consumer Affairs establishes norms that define the minimum standards and requirements for health care provision. These are (as a minimum) equal for all Spanish citizens.

An important role in the Spanish national health system is played by the Interterritorial Council of the National Health System (Consejo Interterritorial de salud), which guarantees a minimum of coordination between the regions and the national level. Since the Law on Cohesion and Quality of 2003 [47] the council is composed of the National Minister of Health and Consumer Affairs and the Ministers of all the Autonomous Communities that are responsible for health issues at regional level. It coordinates, sets up information systems and assures cooperation between the national and the regional health authorities. In the context of the decentralized health care system, the Law on Cohesion and Quality outlines the framework of cooperation between public administration departments and proposes significant future reforms. A number of strategies, such as the establishment of the NHS Agency on Quality, the Health Information Institute and the National Observatory of the NHS, have already been implemented.

Central government has the responsibility for promoting coordination and cooperation in the health sector. It assumes responsibility for certain strategic areas, including:

- general coordination and basic health legislation;
- financing of the system, and regulating the financial aspects of social security;
- definition of a (minimum) benefits package guaranteed by the NHS;
- international health;
- pharmaceutical policy.

The regions are responsible for:

- Health planning;
- Public health;
- Care delivery.

The Communidades Autonomas are responsible for the organization of care. Financial means (mainly tax income) are transferred to the regions and the regions manage, control and organize the care delivery system. They are responsible for the organization of primary care, specialist care (hospital level), pharmaceutical services and emergency care in their region. The regions (through the regional health authorities) that manage health care provision and health care institutions. As such every region has the power to expand care delivery and provide additional care and coverage next to the minimum benefits package that has to be guaranteed (determined at national level).

The possibility for the regions to have and to organise additional care provision means, the regions can include other medical services, treatments, drugs, medical devices, home care…. in the regional public health system. As a consequence, one can observe sometime substantial differences in care provision to the citizens depending from the priorities defined by the regional health authority from the region they live in.

9.16.2.1 Who is covered?

In Spain, one should distinguish between:

The National Health system (public health insurance)

The NHS, through the Regional Health Services, provides universal health care to all of its citizens. The universal basic health insurance provides a standard benefits package for all residents. Over 90% of the population uses the National Health System for its medical needs.

Health care provision in Spain mainly is organised at two levels: The primary care level and the specialist care level (hospital care). It allows a citizen to choose his primary care doctor being a part of the NHS system. The general practitioner has a very important role in the NHS. It's through the GP the citizens access the rest of the system. Primary care is mostly concentrated in the primary health care centres that provide the GP services, paediatrics and nursing care. The primary care centres also provide midwives, physiotherapists and social workers. In order to consult a specialist, patients must first be referred by their primary care doctor (except in emergencies). Specialists mostly work in the hospitals that operate within the NHS system. Most of these hospitals are owned by the Regional Health Services. Otherwise it can be private hospitals that are recognised and have an agreement with the Regional Health Service. There are generally no out-of-pocket expenses for the medical care at primary or secondary care level as the Regional Health Services pay for all medical care.

Complementary private health insurance:

In Spain the last decade, the private health care sector became more important. About 15% of the population holds private health insurance including most civil servants who have the choice of coverage by the public or the private systems. The private health insurance is used either as a supplement or an alternative to public care.

The private insurance companies have their own network of hospitals, clinics and laboratories. Policyholders usually do not have to wait as long for treatment as patients using the NHS. At secondary care level, within the NHS, waiting lists are an important issue. The private insurance companies can direct their members to doctors who are members of their group. However, most of these private insurers have programs that refund 80% of the fees charged by physicians outside the group. Adeslas, Asisa and Sanitas are the largest private health care providers.

9.16.2.2 What is covered?

The 63/1995 Royal Decree for Services Provision[48] drew up a list of medical services guaranteed by the public health system (maintaining those already in existence and including those not available to all inhabitants under universal coverage) while trying to apply security, effectiveness, quality and efficiency criteria to control those newly introduced services and technologies.[49] It defined benefits as detailed below. Primary health care includes general medical and pediatric health care provided at health centers and during home visits, programmes for prevention of disease, health promotion, and rehabilitation. Specialized health care in the form of outpatient and inpatient care covers all medical and surgical specialties in acute care.

In 2006 a new Royal decree modified the minimal health care services to be guaranteed to all citizens. Access to NHS health care provision is regulated in the 'Cartera de servicios'. This is a kind of minimum list of common services to be provided and ensured to all citizens regardless of where in the national territory they live or reside.

9.16.2.3 Service portfolio (Cartera de servicios)

The definition and update of the common service portfolio will take into account the safety, effectiveness, efficiency, effectiveness and usefulness of therapeutic techniques, technologies and procedures as well as the advantages and alternative care. Prior to his inclusion in the 'cartera de servicios' of techniques, technologies or processes for patient treatment and products are assessed. It is imperative that:

The drugs are approved for commercialization and are used according to the specifications as foreseen in the technical fiche

Medical devices, including implants and diagnostic reagents in vitro, have the CE mark for the indication in question, and meet the requirements established by the Royal Decree which regulates medical devices.[50]

Incorporation of new techniques, technologies or procedures to the 'cartera de servicios' requires evaluation by the Ministry of Health and Consumer Affairs through the agency of health technology assessment of the Carlos III Health Institute (in collaboration with the other evaluation organizations proposed by the Autonomous communities)..

New techniques or procedures have to demonstrate at least one of the following characteristics:

- Represent a substantially innovative contribution to the prevention, the diagnosis, the therapeutics, rehabilitation, improvement of life expectancy or the elimination of pain and suffering.
- Being new indications for already existing devices or products
- Requiring new specific devices or techniques
- Modify significantly the forms or organizational systems of care to patients
- Have an effect on large populations or risk groups
- Assume a significant economic impact in the NHS
- Assume a risk for the patients or health professionals

A proposed update can be initiated by the health administrations of the regions or of the Ministry of Health and Consumer Affairs through the Interterritorial Council of the National Health System. A special committee (Comisión de prestaciones, aseguramiento y financiación) agrees on the proposal on the inclusion or exclusion of the technique, the technology or the process concerned. Final approval of the proposals put forward by the commission, is done by the Ministry of Health and Consumer affairs, in agreement with the Interterritorial Council of the National Health System.

9.16.2.4 Additional services portfolio of the autonomous communities:

The autonomous communities, within the scope of their powers, shall adopt their respective portfolios of services, including, at least, the portfolio of common services of the National Health System, which should be available to all users. The autonomous communities may incorporate in their portfolios of services, a technique, technology or procedure not covered in the portfolio of common services of the National Health System. The regional health authority can established additional requirements. In any case, these additional services must meet the same requirements set for acceptance in the NHS. These additional services will NOT be included in the overall financing of benefits under the National Health System. This means these services will have to be paid for by the Regional authorities.

9.16.2.5 Medication

For medication (drugs and other sanitarian products) a specific portfolio of products has been foreseen for the NHS. (Cartera de servicios comunes de prestación farmacéutica)

It contains the pharmaceutical drugs and other products and sets the conditions on which patients receive them appropriated to their clinical needs, in measured doses according to their individual requirements, during the appropriate time and at the lowest possible cost to themselves and the community. The provision of drugs and products is regulated by the Law 29/2006 of 26 July, on guarantees and rational use of medicines and medical devices and other applicable provisions.[51]

The 'cartera de servicios de prestación farmacéutica' differentiates the medication for patients that are treated in the specialized care (hospital care) and those that are not (primary care).

For patients outside the hospital care, the 'cartera' comprehends the prescription and the provision of drugs within the NHS. Products have to be authorized and registered by the Agencia Española de Medicamentos y Productos Sanitarios or have to meet EU regulation on autorisation and control of medical drugs for human use. These drugs and care products are distributed through local pharmacies.

For the drugs delivered at hospital care level the 'cartera de servicios communes de atención especializada ' contains the pharmaceutical products to be used and financed by the NHS. Of course this portfolio (cartera) can also be expanded by the regional health authorities. It sets the indications and the administration of medication parenteral or enteral nutrition, cures, consumables and other health products. This 'cartera' also contains the implants and medical devices used at hospital level.

9.16.2.6 Acceptance of drugs in the NHS system

The decisions to integrate new medication in the NHS (minimum to be guaranteed for all citizens) are taken by the Ministry of Health in accordance with the orientations of the 'Consejo Interterritorial del Sistema Nacional de Salud'.

New drugs are to be approved and registered by the AEMPS (Agencia Española de Medicamentos y Productos Sanitarios). Once they are, the Ministry of Health and Consumer Affairs will decide by a reasoned decision, prior to placing the drug on the market, to include or not the drug in the National Health System. In case of inclusion it will also decide on the conditions of this inclusion. Price setting is done by a specific committee, the 'Comisión Interministerial de Precios de los Medicamentos'.

The AEMPS has different functions related to the drug policy. The AEMPS holds the competences to:

- Grant marketing authorization for pharmaceuticals and other medicines for human use manufactured industrially, as well as to review and make appropriate adjustments in the existing market.
- Participate in the planning and evaluation of medicinal products for human use that are accepted by the EU through the European Agency for the Evaluation of Medicinal Products.
- Evaluate and authorize trials and products under clinical investigation.
- Authorize pharmaceutical drugs for human use.
- Plan, assess and develop the Spanish pharmaco vigilance system.
- Develop inspection activity and drug control state competition.
- Manage the Royal Spanish Pharmacopoeia.
- Instruct the procedures associated with drug-related offenses as appropriate to the General State Administration.
- Take responsibility for narcotic drugs and psychotropic prescribed by regulation.

9.16.2.7 Costs of medical treatments and drug provision payment

There is no cost sharing in access to primary or specialist care services in the public sector. Hospitals in the National Health System are funded through a global budget granted by the Regional Health Authorities.

Medication is provided by local pharmacies (ambulatory care) and hospital pharmacies (hospital care and ambulatory hospital care provided by the medical specialists).

For medication that is purchased at the local pharmacies there is a system of co-payment. There are mechanisms to protect vulnerable groups of people.

- For people under 65 years of age who do not suffer from permanent disability or chronic illness, 40% co-payment of the official price is required
- For drugs for chronic diseases or serious diseases , (expanded to AIDS patients) the co-payment level is set at only 10% of the cost with a maximum of 2,64 € per drug prescribed (this amount can be actualized by the Minister of health)
- All users of the civil servants' mutual funds only pay 30% of pharmaceutical costs
- Some groups of patients as pensioners, patients with disabilities, work accidents and occupational disease don't have to contribute at all. They are excluded from co-payment.

Products that are provided to the patient in the primary health care centers are also excluded from co-payment. For medication provided at hospital level there is no co-payment. They are part of the hospital budget.

Cost sharing in the area of pharmaceuticals, as well as medical aids and prostheses (including hearing aids and corrective lenses) has been significant since the late 1990s. At national level a decision has been taken (within the Interterritorial Council of the NHS) that there should be no regional variations in cost sharing. However, since the regions have introduced some changes in the coverage of services and entitlements over the last years, it is a reality some differences exist.

Complementary benefits include prostheses, orthopedic products, wheelchairs, health care transportation, complex diets and home-based oxygen therapy. Recently, children's hearing aids were also included in this package. In general, the user does not pay for these complementary benefits, except for certain orthopedic products or prostheses.

Exclusion criteria for other benefits not covered by the public system include:

Lack of evidence on safety or clinical effectiveness, or evidence that the intervention has been made redundant by other available procedures (for example, a negative list of pharmaceuticals was introduced for the first time in 1993 and updated in 1998 excluding all products of unproven clinical effectiveness from public funding); classification of the intervention as a leisure activity, relating primarily to rest and comfort (e.g. sports, aesthetic or cosmetic improvement, water therapy, residential centers..).

9.17 SSF CASES FOR INTERNATIONAL BENCHMARKING

9.17.1 The Netherlands

Name	Active substance	Indication	Reimbursement	Source	Citation of the expert / Rekevant remark
Adagen	Pegademase bovine	A type of SCID (severe combined immunodeficiency syndrome) caused by the chronic deficiency of the adenosine deaminase (ADA) enzyme	US-registered orphan drugs (non-EU): not reimbursed intra muros, nor extra muros. Today, adagen could possibly be paid by pharmacist's, pharma company or the hospital through ad hoc-reimbursement systems (however not by the health insurers) GREY ZONE.	J.Huizer, Dutch Steering Committee on Orphan Drugs	With respect to reimbursement registered drugs are granted priority over non-registered (in NL). In the NL there is no special legislation for the reimbursement of non-registered drugs. A request for advice for the treatment and reimbursement can be submitted at the Federation of Health Insurers (College voor Zorgverzekeringen). The Health insurer will then take care of the reimbursement. However, the health insurer can also decide not to accept the advice of the CVZ.
Vidaza	Azacitidine	Myelodysplastic syndrome	Received recently a positive advice to be recognized as 'Expensive Drugs' (intra muros) - 80% reimbursable - prescribed by specialists in all hospitals	J.Huizer, Dutch Steering Committee on Orphan Drugs	If In case of intramural treatment, drugs that are listed under the policy regulation 'Expensive drugs' are being compensated by the state to hospitals for 80%. The other 20% share is charged on the hospital budget. Drugs that are listed under the policy regulation 'orphan drugs' are fully (100%) compensated to the hospital (however, only academic hospitals).
Kuvan	sapropterin dihydrochloride	Phenylketonuria (PKU) - tetrahydrobiopterin defiency	Received recently a positive advice to be adopted in the Drug Reimbursement System (GVS-extra muros) - 100% reimbursable	J.Huizer, Dutch Steering Committee on Orphan Drugs	No information available.
Cellcept	Mycophenolate mofetil	Auto immunology diseases - neophrotic syndrome	Reimbursed under GVS - Schedule 2 (advies CVZ, 2007)	A. Prenger, Zorgverzekeraars Nederland+literature review	A positive advice has been given for the reimbursement, in case of treatment of neophrotic syndrome.
Mabthera	Rituximab	Heamatologic oncology (leukemia - lymphoma)	Recognized as 'Expensive Drugs' (intra muros) - 80% compensated - prescribed by specialists in all hospitals	A. Prenger, Zorgverzekeraars Nederland+literature review	Listed under the policy regulation 'Expensive drugs': 80% compensation for hospitals
Avastin	Bevacizumab	Brain tumors (glioblastoma) - eye disorders-cancer colon métatasé 2ième intention-tumeur de l'ovaire	Recognized as 'Expensive Drugs' (intra muros) - 80% reimbursable - prescribed by specialists in all hospitals	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	Listed under the policy regulation 'expensive drugs for a number of indications. However, no information could be found concerning the reimbursement in case of brain tumor or eye cancer. Hospitals are probably compensated for 80%. 20% is charged on the hospital budget.
Bi-myconase and/or sucraid	Glucamylase & invertase (saccharase)	Enzyme deficiency sucrose isomaltase	Not reimbursable by the Health Insurer (high probability)	J.Huizer, Dutch Steering Committee on Orphan Drugs	Since 1995, glucoamylase is excluded from the treatment for acute diarrhoea. Enzym deficientie sucrose isomaltase is not on the list of the patient organisation for metabolic indications. Probably not reimbursed by the health insurer.
Ointmenst, disinfectants, painkillers, tape, food supplements		Epidermolysis bullosa - ichthyosis			No information.
Implantable cardiac valves (aortic valves)		Degenerative valve disease (aged persons)			No information.
Implantable pulmonary valves		Congenital cardiac malformations (children)			No information.
Brainstem implant		Patients who have no remaining auditory nerves (ex. Bilateral vestibular schwannomas tumors on both balance nerves)	Probably not reimbursed under GVS - Advies CVZ	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	
Neuro stimulator of the stomach		Severe gastro paresis	Hospitals are probably compensated to a specific limit ((only for academic hospitals), as the Neurostimulator is listed under a policy regulation	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	Neurostimulator: no information on current status. However, it seems that hospitals are partly compensated for the costs (up to a specific maximum), as the neurostimulator has been the subject of a policy regulation that only applies to academic hospitals.

Name	Active substance	Indication	Reimbursement	Source	Citation of the expert / Rekevant remark
Remodulin	tréprostinil	pulmonary hypertension	Reimbursed under GVS - listed under Schedule 2 + Paid on the hospital budget (intra muros)	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	
Viagra/revatio		pulmonary hypertension	Reimbursed under GVS - listed under Schedule 2 + Paid on the hospital budget (intra muros)	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	No information.
Ilomedine	iloprost	pulmonary hypertension	Reimbursed under GVS - listed under Schedule 2 + Paid on the hospital budget (intra muros)	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	No information.
Elvorine		cerebral folate deficiency	Not registered in NL - no request for marketing authorisation could be found	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	Not registered in the NL and no request for reimbursement has been submitted. No further information available.
Calcort	déflazacort	duchenne muscular dystrophy-syndrome néphrotique	Not registered in NL - not reimbursed. Patients/parents are paying it out-of-pocket.		Not registered in the NL and not reimbursable. Costs are charged on the patients.
Myozine	alglucosidase alfa	Pompe disease	Paid on the hospital budget (intra muros): adopted on the list of 'Orphan Drugs' - 80 % reimbursed - only prescribed by specialists in the 8 academic hospitals	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	No information.
Sovrima	idebenone	Friedreic's ataxia	Not reimbursed under GVS - Still in clinical trial process - Patients/parents are paying it out-of-pocket	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	Not registered yet (procedure is ongoing at EMEA level) - not reimbursable. Costs are charged on the patients.
Ditropan for intravesical application	oxybutynin chloride	incontinency neurogenic bladder			No information.
Cochlear, contraleteral implant		Sensori-neural hearing loss			No information.
Customised cranial implant		"lack" of bones of the skull (after accident, operation, malformation,,,)			No information.
Cortical electrodes		mise au point en vue d'envisager une intervention au niveau de l'encéphale en cas d'épilepsie réfractaire			No information.
Intégra		in case of skin graft as a consequence of a fire, suite à des brûlres, diverse impairments (accidents…)			No information.
Middle ear implant		Sensori-neural hearing loss			No information.
Diafragmatic pacemaker		diafragmatic paralysis	No information could be found. Possibly it is paid by the hospital budget.	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	No information. Possibly charged on the hospital budget.
Contrathion			No information could be found. Possibly it is paid by the hospital budget.	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	No information.
Cystadane		homocystinuria	Reimbursed under GVS - listed under Schedule 1B - 100% reimbursable	J.Huizer, Dutch Steering Committee on Orphan Drugs + Literature review	

9.17.2 France

The assessment of the cases is based on telephone interviews with experts and desk research. The desk research consisted from an in-depth analysis of the Nomenclature database, available at www.ameli.fr. This database contains all products and services that are reimbursed under the Assurance Maladie Obligatoire.

The Excel sheet uses the Codification language which is used by the French nomenclature of Medicines, Products and Services. The nomenclature distinguishes between:

- **CIP codes (Club Inter Pharmaceutique)**, which reflect the Marketing Authorisation of a specific medicine and ensure the traceability of drugs. CIP codes, consisting from 7 numbers, are administered by the Club Inter Pharmaceutique and the AFSSAPS.

CIP codes can be further split up between:

  - Drugs, for which only so-called *collectivités* (hospitals, residential homes for the elderly…) are recognized agents;
  - Drugs, that are *remboursable aux assurés sociaux and/or agree aux collectivités.*

- UCD codes (Communes de Dispensation Utilisées à l'hôpital) are only attributed to medicines that are used within hospitals or other health institutions. The UCD codification is still in use as the exchange standard regarding reimbursements of services (tarification à l'activité, T2A) and so-called rétrocession (medicines that are prescribed and purchased intra muros for patients that are not hospitalized). In case of rétrocession, the costs of drugs are not charged on the hospital budget. Also UCD codes are being developed and administered by the CIP.

Name	Active substance	Indication	CIP	UCD	Source
Adagen	Pegademase bovine	a type of SCID (severe combined immunodeficiency syndrome) caused by the chronic deficiency of the adenosine deaminase (ADA) enzyme	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie
Vidaza	Azacitidine	myelodysplastic syndrome	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités: sinds 01/2009	UCD: Rétrocession: 100% - sinds 18/02/2009 - Médicament Non facturable en sus de la TAA	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie
Kuvan	sapropterin dihydrochloride	phenylketonuria (PKU) - tetrahydrobiopterin defiency	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités: sinds 04/2009	UCD: Rétrocession: 65% (sinds 24/04/2009) - Médicament Non facturable en sus de la TAA	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
Cellcept	Mycophenolate mofetil	auto immunology diseases - neophrotic syndrome-greffe pulmonaire-greffe des ilots de Langhermans	CIP: depending on the product; Homologation Assurés Sociaux (A) - 100% - since 10/01/2006 (re-examined in 2013) + Homologation Collectivité C: since 10/01/2006	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
Mabthera	Rituximab	Heamatologic oncology (leukemia - lymphoma)	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités C: since 1998	UCD: Rétrocession: 100% (since 11/05/2005) - TAA: 11/05/2005	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
Avastin	Bevacizumab	brain tumors (glioblastoma) - eye disorders-cancer colon métatasé 2ième intention-tumeur de l'ovaire	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités C: sinds 07/2005	UCD: Médicament Non Remboursable au titre de la rétrocession - TAA: 09/2005	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
Bi-myconase and/or sucraid	glucamylase & invertase (saccharase)	enzyme deficiency sucrose isomaltase	Not found in the nomenclature	Not found in the nomenclature	No information found
Ointmenst, disinfectants, painkillers, tape, food supplements		epidermolysis bullosa - ichthyosis	Not found in the nomenclature	Not found in the nomenclature	No information found
Implantable cardiac valves (aortic valves)		degenerative valve disease (aged persons)	Not found in the nomenclature	Not found in the nomenclature	No information found
Implantable pulmonary valves		congenital cardiac malformations (children)	Not found in the nomenclature	Not found in the nomenclature	No information found
Brainstem implant		Patients who have no remaining auditory nerves (ex. Bilateral vestibular schwannomas tumors on both balance nerves)	Not found in the nomenclature	Not found in the nomenclature	No information found
Neuro stimulator of the stomach		Severe gastro paresis	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Nomenclature Assurance Maladie: Liste des prestations et des Services (LPP)
Flolan	époprosténol	pulmonary hypertension	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités	UCD: Rétrocession: 100% (sinds 1/05/2004) - TAA: 05/2005	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)

Legende
CIP (Club Inter Pharmaceutique): remboursables aux assurés sociaux et agréés aux collectivités (maisons de rétraites, hôpitaux)
UCD (unités communes de dispensation): rétrocédés et facturables en sus des GHS par les établissements de santé

Name	Active substance	Indication	CIP	UCD	Source
Remodulin	tréprostinil	pulmonary hypertension	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités: sinds 10/2005	UCD: Rétrocession: 100% (sinds 31/12/2005) - TAA: 01/04/2006	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
Viagra/revatio		pulmonary hypertension	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités: sinds 04/2006	UCD: Rétrocession: 100% (sinds 09/2006) - Médicament Non facturable en sus de la TAA	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
Ilomedine	iloprost	pulmonary hypertension	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) / Agrées aux collectivités: sinds 1992	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	
Elvorine		cerebral folate deficiency	Not found in the nomenclature	Not found in the nomenclature	No information found
Calcort	déflazacort	duchenne muscular dystrophy- syndrome néphrotique	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
Myozine	alglucosidase alfa	Pompe disease	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
Sovrima	idebenone	Friedreic's ataxia	Not found in the nomenclature	Not found in the nomenclature	No information found
Ditropan for intravesical application	oxybutynin chloride	incontinency neurogenic bladder	CIP: depending on the product; Homologation Assurés Sociaux (A) - 100% - sinds 10/01/2006 (reexamen in 2013) / Agrées aux collectivités: sinds 06/2006	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
implant cochléaire contralétéral (donc en fait bilatéral)		Sensori-neural hearing loss	Not found in the nomenclature	Not found in the nomenclature	No information found
implant crânien sur mesure		"lack" of bones of the skull (after accident, operation, malformation,,,)	Not found in the nomenclature	Not found in the nomenclature	No information found
électrodes corticales		mise au point en vue d'envisager une intervention au niveau de l'encéphale en cas d'épilepsie réfractaire	Not found in the nomenclature	Not found in the nomenclature	No information found
intégra		in case of skin graft as a consequence of a fire, suite à des brûlres, diverse impairments (accidents…)	Not found in the nomenclature	Not found in the nomenclature	No information found
implant de l'oreille moyenne		Sensori-neural hearing loss	Not found in the nomenclature	Not found in the nomenclature	No information found
pace maker diaphragmatique		diafragmatic paralysis	Not found in the nomenclature	Not found in the nomenclature	No information found
contrathion			CIP: Médicament NON Remboursable aux Assurés Sociaux (A) wel Homologation Collectivité C sinds 1995	Not found= not reimbursed under the statutory health insurance (l'Assurance Maladie obligatoire)	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)
cystadane		homocystinuria	CIP: Médicament NON Remboursable aux Assurés Sociaux (A) wel Homologation Collectivité C sinds 04/2008	UCD: Rétrocession: 65% (sinds 04/2008) - Médicament Non facturable en sus de la TAA	Mrs. Isabelle Chenney + Nomenclature Assurance Maladie (Base des médicament et informations tarifaires)

9.17.3 Spain

Name	Activ sustance	Indication	Reimbursement
Adagen	Pegademase bovine	a type of SCID (severe combined immunodeficiency syndrome) caused by the chronic deficiency of the adenosine deaminase (ADA) enzyme	
Vidaza	Azacitidine	myelodysplastic syndrome	Hospital use Financed NHS without any co-payment of the patient
Kuvan	sapropterin dihydrochloride	phenylketonuria (PKU) - tetrahydrobiopterin defiency	Hospital use Financed NHS without any co-payment of the patient
Cellcept	Mycophenolate mofetil	auto immunology diseases - neophrotic syndrome	Diagnose by medical specialist Co-payment of the patient 10% max.2,64€
Mabthera	Rituximab	Heamatologic oncology (leukemia - lymphoma)	Hospital use Financed NHS without any co-payment of the patient
Avastin	Bevacizumab	brain tumors (glioblastoma) - eye disorders-cancer colon métatasé 2ième intention-tumeur de l'ovaire	Hospital use Financed NHS without any co-payment of the patient
Bi-myconase and/or sucraid	glucamylase & invertase (saccharase)	enzyme deficiency sucrose isomaltase	
Ointmenst, disinfectants, painkillers, tape, food supplements		epidermolysis bullosa - ichthyosis	
Implantable cardiac valves (aortic valves)		degenerative valve disease (aged persons)	In hospital's budget
Implantable pulmonary valves		congenital cardiac malformations (children)	In hospital's budget
Brainstem implant		Patients who have no remaining auditory nerves (ex. Bilateral vestibular schwannomas tumors on both balance nerves)	In hospital's budget
Neuro stimulator of the stomach		Severe gastro paresis	
Flolan	époprosténol	pulmonary hypertension	Hospital use Financed NHS without any co-payment of the patient
Remodulin	tréprostinil	pulmonary hypertension	
Viagra/revatio		pulmonary hypertension	Hospital use Financed NHS without any co-payment of the patient
Ilomedine	iloprost	pulmonary hypertension	Hospital use Financed NHS without any co-payment of the patient
Elvorine		cerebral folate deficiency	
Calcort	déflazacort	duchenne muscular dystrophy- syndrome néphrotique	Other product - on prescription for long time treatment co-payment of 40%
Myozine	alglucosidase alfa	Pompe disease	Hospital use Financed NHS without any co-payment of the patient
Sovrima	idebenone	Friedreic's ataxia	
Ditropan for intravesical application	oxybutynin chloride	incontinency neurogenic bladder	In hospital's budget
implant cochléaire contralétéral (in fact bilatéral)		hypoacusia	In hospital's budget
Cranial implant on messure		shortage of cranial bone (after accident, operation or malformation)	In hospital's budget
corticale electrodes		in case of refractive epilepsy awaiting surgery at encefale level	In hospital's budget
intégra		skin substitute that regenerates dermis burn treatment	
implant in mid ear		hypoacusia	In hospital's budget
diafragmatic pacemaker		Diaphragmatic Paralysis	In hospital's budget
contrathion			
cystadane		homocystinury	Hospital use Financed NHS without any co-payment of the patient

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