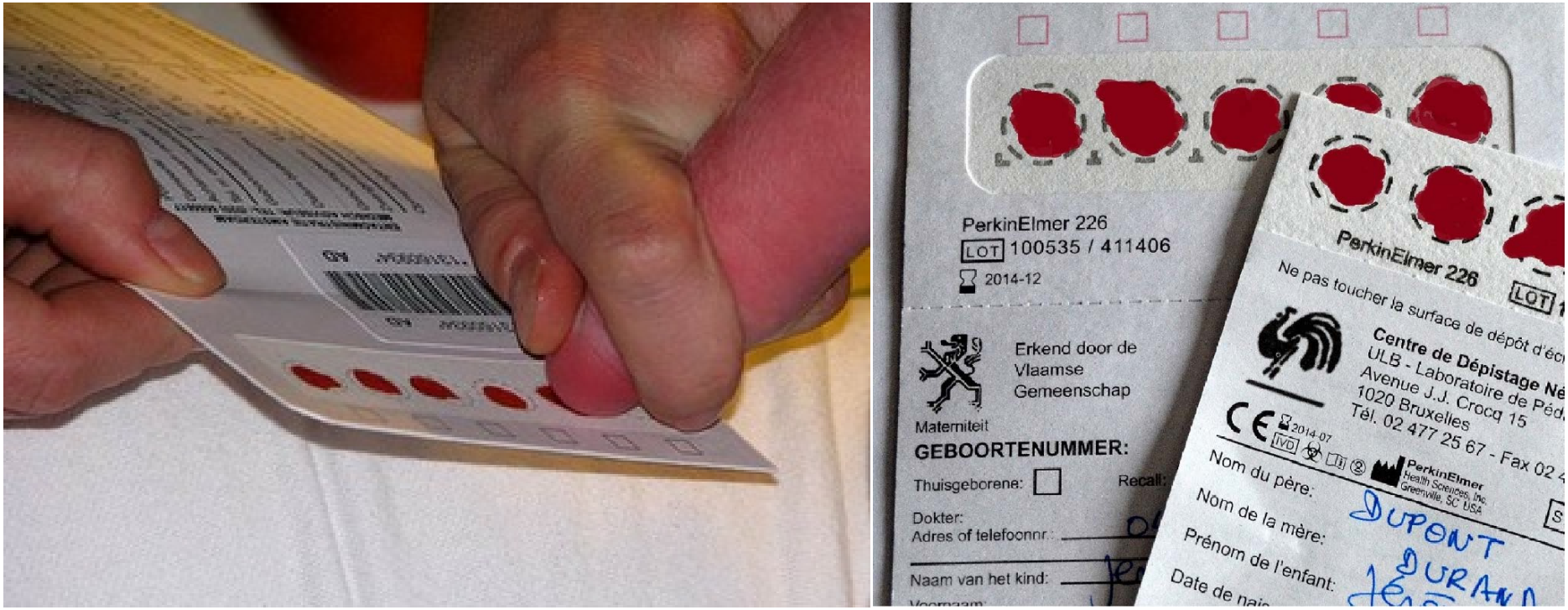
KCE REPORT 267Cs

Federaal Kenniscentrum voor de Gezondheidszorg
Centre Fédéral d'Expertise des Soins de Santé
Belgian Health Care Knowledge Centre

SYNTHESIS

MULTI CRITERIA DECISION ANALYSIS TO SELECT PRIORITY DISEASES FOR NEWBORN BLOOD SCREENING



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HEALTH SERVICES RESEARCH

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SYNTHESIS

MULTI CRITERIA DECISION ANALYSIS TO SELECT PRIORITY DISEASES FOR NEWBORN BLOOD SCREENING

CHRIS DE LAET, GERMAINE HANQUET, ERIK HENDRICKX

FOREWORD

In Europe in 2016, we might reasonably hope that healthcare had little by little reached a certain degree of harmonisation. Do we not have, for each discipline, 'European Societies' that conscientiously produce recommendations for good practices and consensuses, all based in theory on the same evidence-based international sources? Yet with regard to screening for metabolic illnesses in newborns, nothing could be further from the truth. Depending on the country where the little European is born, his heel prick will involve one, two… or thirty diseases to be tested. It is true that all Europeans do not come from comparable genetic pools; undoubtedly it would not make much sense to screen for thalassaemia in Lapland… although with the waves of migrations that we are experiencing, the situation could well become more complicated. But even between countries – or regions¬ ¬– that screen for a comparable number of illnesses, the composition of the analysis panels can vary considerably.

The choice of an 'optimal' analysis panel is of course a complex issue in which a whole series of aspects must be balanced. And we are all familiar with the committee meetings where this type of complex tangle must be unravelled: the discussions that jump constantly from one aspect of the problem to another, the opinion leaders who monopolise the discussion, all of the parties involved who try to assert their own perspectives, to the point that ultimately no one can keep an overall view of the issue. And this invariably ends with different lists for each country or region, always with good reasons, but often conditionally and not always consistently.

Here and there the idea is gaining ground that decisions like those on neonatal screening could be made in a more coherent way, and on this point we have found the example of Québec very inspiring. In the study in front of you, we have therefore attempted to evaluate the degree to which their model of multi-criteria decision analysis (MCDA) is applicable to Belgium (read: to the Walloon, Flemish and Brussels realities). This is the second time that we are undertaking this exercise in the context of the Belgian healthcare system; we are also involved in testing a MCDA model with the NIDHI [National Institute for Health and Disability Insurance] in connection with the issue of unmet medical needs.

We may hope to see here the beginnings of a fundamental cultural turning point that, with the aid of such tools, will venture to make decisions in such complex areas much more objective and in particular much more transparent.

Christian LÉONARD
Deputy general director

Raf MERTENS
General director

SYNTHESIS

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1. NEWBORN BLOOD SCREENING (NBS) PROGRAMMES

A Newborn Blood Screening (NBS) programme is a public health programme intended to systematically screen all infants shortly after birth for a list of conditions that are treatable, but not clinically evident in the newborn period. Most diseases included in NBS programmes are inborn metabolic diseases whose first symptoms appear during the neonatal period or early childhood, after irreversible damage has occurred. Screening for these diseases before symptoms appear allows for an early diagnosis and early interventions. Neonatal screening is also commonly called the neonatal heel prick or Guthrie test. A small blood sample is taken and the blood is soaked into a pre-printed collection card (known as Guthrie card).

Internationally, there is no clear consensus on which inborn diseases need to be screened. As a result, the number and nature of included diseases varies enormously by country, from none to over forty diseases.

In Belgium, the NBS programme started in 1968 with the systematic screening of all newborns for one metabolic disease (phenylketonuria). Six other diseases have been progressively added to the programme.

In the early eighties, the responsibility of the programme has been transferred to the Communities, the 'Vlaamse Gemeenschap' (VG) and the French speaking community (later called Fédération Wallonie-Bruxelles" (FWB)).[a] Since then, the list of diseases to be screened is decided by each community, according to their own legal criteria and upon guidance from their own steering committees. As a result, the two communities do not screen for the same set of diseases: currently 11 diseases are screened for in VG and 13 in FWB; 9 of these diseases are screened for in both communities (Table 1).

Both communities use similar technologies for screening, but tests are conducted in separate reference centres (three in FWB and two in VG). Most inborn metabolic diseases (but not all) are today detected by tandem mass spectrometry (MS/MS). Given sufficiently high volumes, this is a relatively cheap laboratory technique allowing to screen for a large number of metabolic disorders simultaneously and rapidly through the analysis of a single blood sample. This screening measures levels of a high number of metabolites and, when the test is positive, it must be followed by confirmatory tests for final diagnosis. In the VG the total cost for the primary screening (including cards, mailing, reporting of results and education material) amounts to around 20 € per newborn.

As the MS/MS technique allows to detect a high number of disorders, programmes must decide which disease should be screened for, aiming at an acceptable balance between benefits and risks. Possible benefits of screening for a disease are that early detection followed by effective intervention can prevent illness, sequelae and in some diseases early death. The main risks are the consequences of false negative and false positive results, involving false reassurance or unnecessary worry and costs respectively.

The aim of this KCE study is to help decision-making on which diseases could be included in the NBS programmes run by the communities.

[a] In FWB, the NBS programme is managed by the 'Office de la Naissance et de L'Enfance' (ONE) since 2015

Table 1 – Diseases included in neonatal blood screening programmes at community level (February 2016)

Disease	Abbreviation	In Vlaamse Gemeenschap	In Fédération Wallonie-Bruxelles	Included in this pilot study
Metabolic disorders				
Biotinidase deficiency	LMCD	Yes	No	Yes
Galactosemia	GAL	No	Yes	Yes
Glutaric acidemia type 1	GA I	Yes	Yes	
Homocystinuria	HCY	No	Yes	Yes
Isovaleric acidemia	IVA	Yes	Yes	
Leucinosis or Maple syrup urine disease	MSUD	Yes	Yes	
Medium chain acyl-CoA dehydrogenase deficiency	MCAD	Yes	Yes	
Methylmalonic acidemia	MMA	Yes	Yes	
Multiple acyl-CoA dehydrogenase deficiency	MADD	Yes	Yes	
Phenylketonuria	PKU	Yes	Yes	
Propionic acidemia	PA	Yes	Yes	
Tyrosinemia Type I	TYR I	No	Yes	Yes
Very long Chain CoA deshydrogenase deficiency	VLCAD	No	Yes	Yes
Endocrine disorders				
Congenital hypothyroidia	CHT	Yes	Yes	
Congenital adrenal hyperplasia	CAH	Yes	No	Yes

2. SCOPE AND METHODOLOGY

In this study, we conduct a pilot testing of a Multi-Criteria Decision Analysis (MCDA) method aiming at disease prioritisation for NBS. This method was applied in Quebec by the Institut National d 'Excellence en Santé et en Services Sociaux (INESSS) in 2013.

In MCDA a number of criteria are selected a priori and attributed a specific weigh. The diseases under assessment are subsequently scored on each of these criteria and ranked based on a global composite score including weight and score for each of the criteria.

The steps in this study are:

1. Select and define in more detail criteria for the evaluation of diseases.
2. Define weights by criterion that is later applied in the calculation of a global composite score by disease. These weights are expressed using a four point LIKERT scale (Table 2).
3. Prepare scientific information for each disease on all criteria in a short disease summary.
4. Score each criterion, by disease. Also for these scores a four point LIKERT scale is used (Table 2).
5. Calculate a global composite score per disease: in general the weight for a criterion is multiplied with the score for a disease for that criterion. This weighted score is than summed per disease, leading to global composite score per disease or an individual composite score for each evaluator. In the last case de scores of the individual evaluators are summed to obtain a global composite score by disease, allowing there ranking.[b]

Table 2 – Description of the LIKERT scales used for scoring criteria weight and disease

Score	Criteria (for weight)	Score of disease for this criterion
1	not relevant	very low
2	slightly relevant	rather low
3	relevant	rather high
4	extremely relevant	very high
0		unable to answer, missing data

Step 1 was conducted jointly by the KCE team together with the steering group consisting of decision makers from both communities, NBS laboratory experts, clinicians, patient representatives and an ethical expert (eleven evaluators in total). Steps 2 and 4 were done exclusively by the evaluators from the steering group while steps 3 and 5 were conducted by the KCE team and later discussed with the steering group.

The steering group and the KCE team jointly decided to select for this pilot study the six diseases that are screened in one community but not in the other (Table 1). Since the scope of this study is pilot testing a method, the results are not recommendations to include or exclude specific diseases from the NBS programs. The application of the methodology and the results allowed to learn a number of lessons that were discussed with the steering group evaluators, and that are described below together with the results.

[b]. Six different methods to calculate global composite score are used; one main analysis and five variants. See scientific report for details.

3. SELECTION AND DEFINITION OF THE CRITERIA

Seven criteria were selected for scoring diseases, inspired by the INESSS methodology (Table 3).

The definitions of these criteria were further refined in the steering group, to match to the highest extent possible the criteria defined in both communities, while limiting the overlap in content. The aim was to avoid that similar aspects of screening would be scored under several criteria, because this could lead to imbalanced end results. The criteria and the description in Table 3 is an abridged version of the kind of data experts wanted in the disease summaries. The original full list can be found in the report.

Table 3 – Description of the criteria used for disease scoring

Criteria	Description
Frequency (birth prevalence)	Birth prevalence in VG, FWB or Belgium if available and/or in Western Europe, North America or world-wide.
Severity of the disease in untreated cases	Severity of the disease without early treatment (natural history), including sequelae, mortality and quality of life loss due to the disease
Timely availability of the test results	Are the test result available at a timely moment to prevent preventable complications and sequelae
Efficacy of early treatment vs. late treatment, according to type of treatment (specific and non-specific)	What is the efficacy of early treatment compared to later treatment What is the evidence that participants (newborns) will probably benefit from this screening and not be harmed by it, for example through early diagnosis of a disease but without an impact on the clinical path or of an untreatable disease that will only become manifest at a later age Degree of consensus about the diagnostic pathway after a test-positive result Degree of consensus about the management of the disease if confirmed Availability of diagnostic and diseased management facilities
Probability and impact of false positive results	Are false positive results reported: what is their frequency and impact?
Probability and impact of false negative results	Are false negative results reported: what is their frequency and impact?
Impact on the health care system	Impact on diagnostic capability and treatment capability, including management of detected (true) case and of side diagnoses Cost of (adding) a specific disease to the existing programme Diagnostic cost for confirmatory tests when screening is positive (both true and false positive) Cost of case management of confirmed disease (care payer and societal) Cost-effectiveness of potential screening for this disease when available Communication towards participants (parents) about potential benefits and harms of screening for additional disease Organizational aspects of screening and its feasibility Acceptability of diagnostic strategy by the population

For two quantitative criteria, ranges of values were pre-defined, to facilitate a homogenous scoring of these criteria across evaluators (Table 4).

Table 4 – Scoring guidance for two quantitative criteria

Score	Disease frequency Birth prevalence (per 100 000 live births)	Timely availability of test results Proportion results available on time to prevent serious complications
1	<1	< 50%
2	1 to 5	50% to <80%
3	>5 to 10	80% to <100%
4	>10 / 100 000	100%

4. DEFINING WEIGHTS OF EACH CRITERION

Each member of the steering group provided a weight for each criterion to reflect its importance in decision making, using a four point LIKERT scale. Preliminary results were then submitted to a steering group meeting and a reweighing was performed after some criteria were clarified and further defined.

The mean and median weight given to each criterion by the evaluators (Figure 1) do not show substantial variations across criteria but a trend for higher weight is observed for the criteria 'disease severity', 'efficacy of early treatment vs. late treatment' and the probability and impact of false negative results (missed cases). However, these measures hide the heterogeneity of the weights given by each evaluator, as shown in Figure 2. The criterion of 'disease severity' was unanimously considered as very important but very divergent answers were observed for the criteria 'disease frequency', 'timely availability of test results and the 'probability and impact of false negative results'. This difference in the evaluation of the criteria weight has been discussed in the steering group and may be due to several factors:

- Although each criterion was defined and detailed, some misunderstanding remained on the precise content of criteria. Members of the steering group proposed that future analyses would be based on a more precise description (e.g. checklist format) of the content of each criterion and in a face-to-face discussion between the evaluators to clarify the content of each criterion.
- The profile of the evaluators likely influenced the allocation of weight given to each criterion. Decision makers, NBS laboratory experts, clinicians, patient representatives and an ethical expert can be expected to have different priorities. For instance the criterion 'timely availability of test results' had a lower importance for some laboratory experts because they automatically adapt the speed of sending the results for a specific disease (for example using the phone rather than mail). Future prioritisation exercises should ensure a balanced distribution of profiles in the evaluation committee.
- The criterion 'impact on the health care system' has a large meaning, involving economic and organisational, feasibility and acceptability

issues, which makes it difficult to weigh (and to score). Moreover, its content is related to both screening and management of detected cases while in Belgium this is organised differently between the two levels of health care: screening policy is part of preventive care and is manage at the level of the Communities, while case management, being part of curative health care, is the competency of the level of the Federal social security (INAMI – RIZIV).

Figure 1 – Weights (means and medians) for the seven criteria (on a LIKERT scale from 1 to 4)

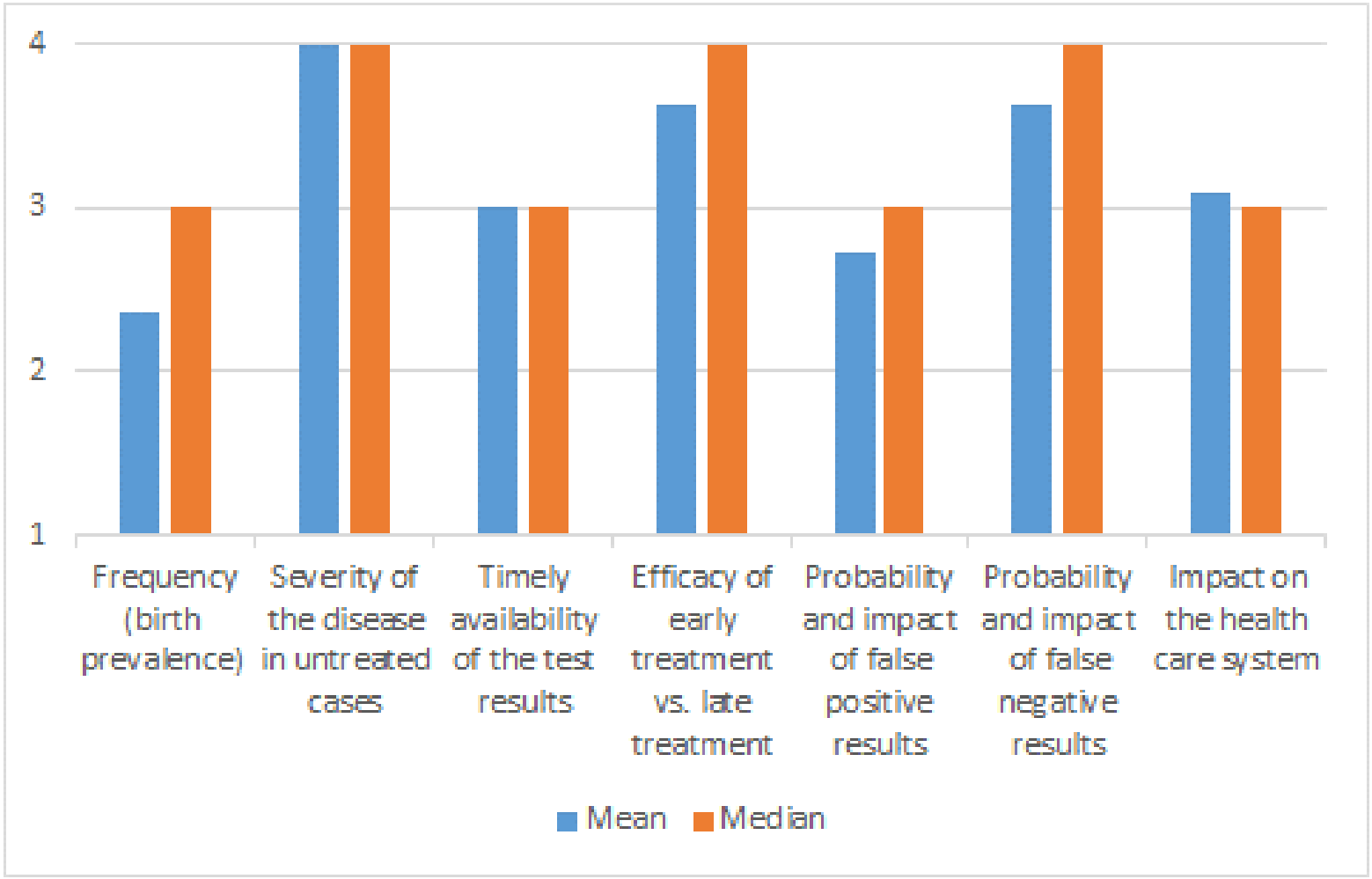
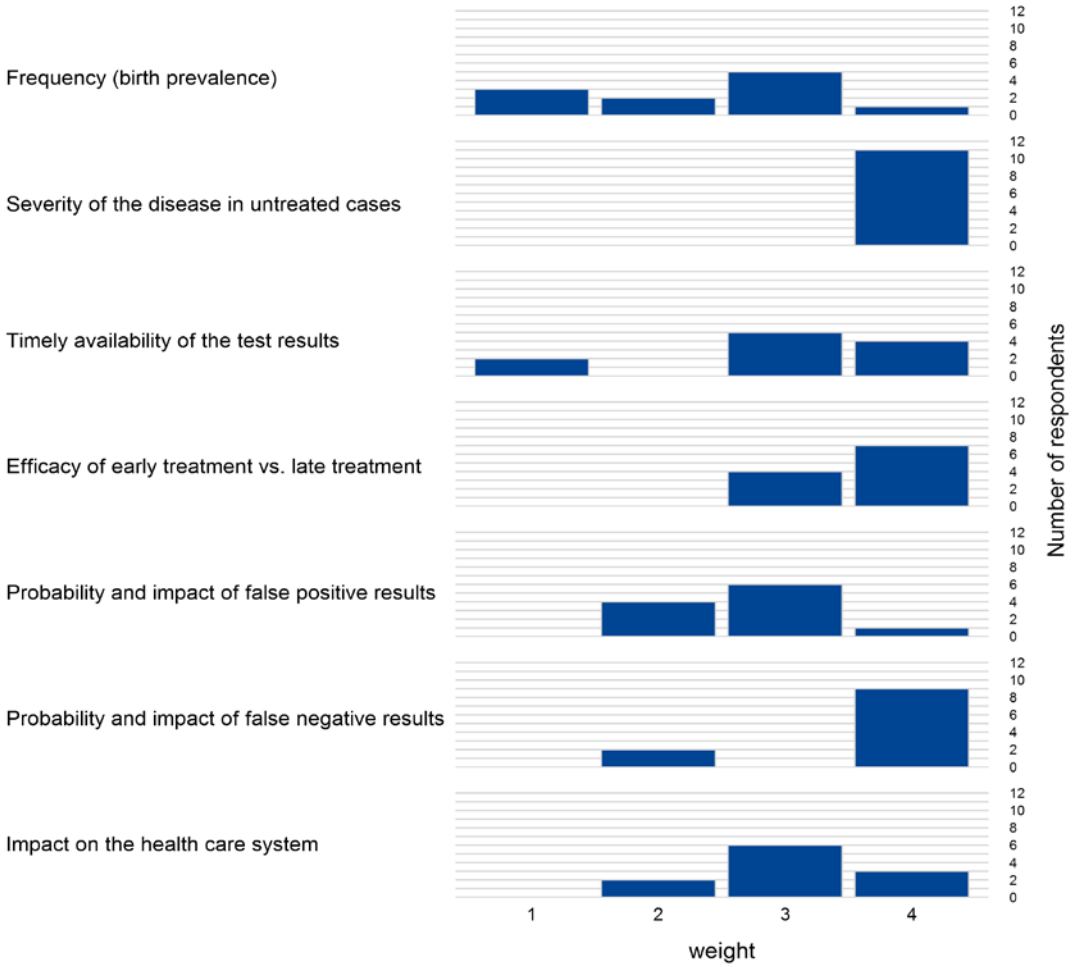


Figure 2 – Distribution of the weight of criteria given by evaluators



5. GATHERING INFORMATION ON EACH DISEASE

For the six selected diseases, information on each criterion was collected by the KCE team through a pragmatic literature review: Belgian data from the communities, information from other agencies (internationally), from evaluators of the steering group, scientific literature and also grey literature. However, full information was not always easily available due to the low frequency of these diseases. Especially on the 'impact on the health care system' information was scarce. This information was synthesised into a disease summary and provided to evaluators as a common and more objective basis for scoring.

6. SCORING BY DISEASE

Evaluators scored the six diseases for each of the seven criteria, leading to a total of 42 disease scores per evaluator. The preliminary scoring results were presented at a meeting of the steering group to discuss their face validity. Criteria and disease information were further clarified and evaluators were able to adapt their scores in case of misunderstandings.

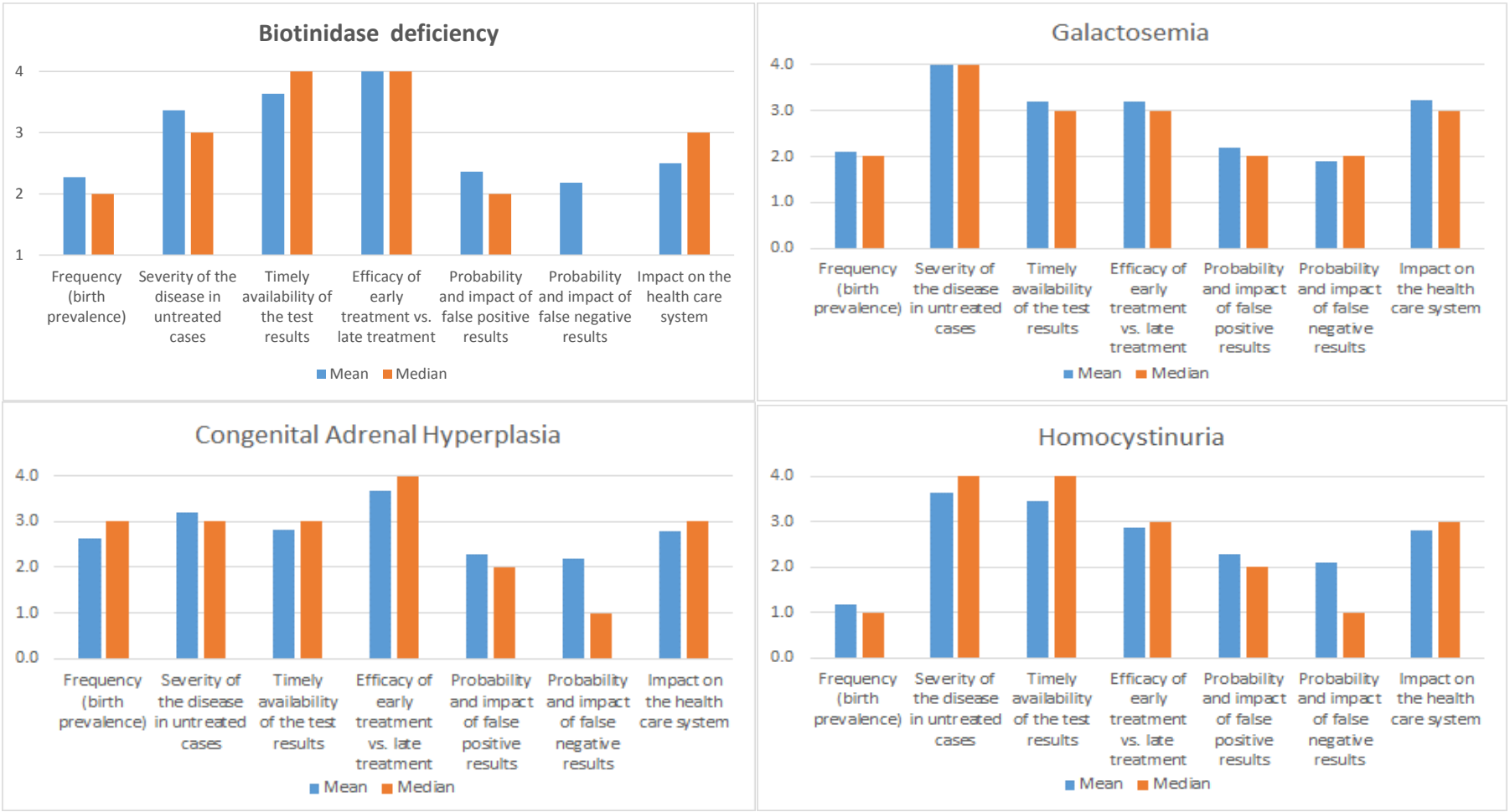
Figure 3 shows the mean and median scores for each criterion (unweighted for criterion weight) for the six diseases. We observe some trends: a high score (median ≥3) is usually given for 'disease severity', 'timely availability of test results' and 'efficacy of early vs. late treatment', while the 'probability and impact of false negative and positive results' generally receive low scores, i.e. median ranging 1-2 (except for TYR I).

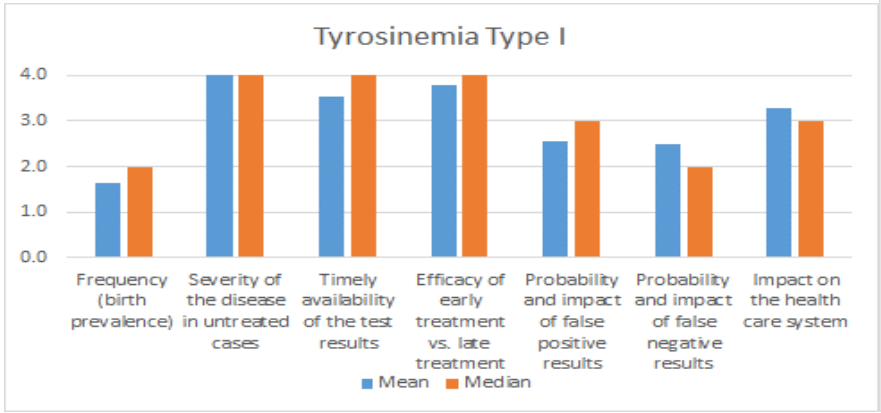
The distribution of evaluator scores by criterion also shows large differences in individual answers, except for the criterion disease severity (more detailed data in scientific report). The largest differences in scores among evaluators are observed for two criteria: the 'probability and impact of false negative results' and the 'impact on the health care system'. Possible reasons for these discrepant scores were discussed in the steering group. This resulted in some hypotheses:

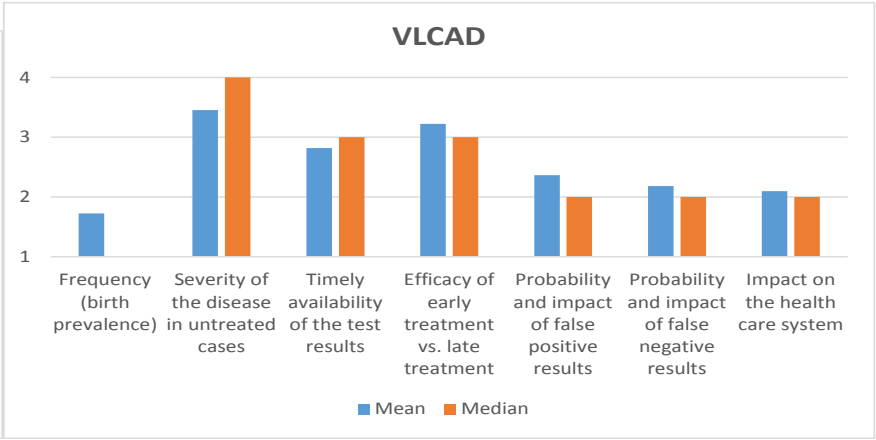
- One explanation is that for some criteria de data are often incomplete since we are dealing with rare diseases. The personal knowledge and experience of evaluators had likely an important influence on the scores in the absence of suffiently robust data.
- For the criterion 'probability and impact of false negative results' a possible explanation was that the probability can by high but the impact low. This makes it difficult to summarise it in a single score.
- The criterion 'impact on the health care system' systematically resulted in dispersed scores (1 to 4 or 2 to 4), which are not reflected by the mean and median scores (i.e. mostly around 3). This was explained by the large definition of this criterion, which contains many different components (Table 3), and to the variation in the profiles of evaluators. For instance evaluators involved in decision making were more sensitive to economic and budget issues than laboratory experts, who gave more importance to the performance of the screening test.

- Decision makers from the communities and evaluators from the NBS labs might be more sensitive to the impact on early detection, while others might be more sensitive to the impact on curative health care.

Figure 3 – Mean and median scores for each criterion by disease (unweighted for criteria importance)







VLCAD: Very long-chain acyl-CoA dehydrogenase

7. COMPOSITE SCORES BY DISEASE

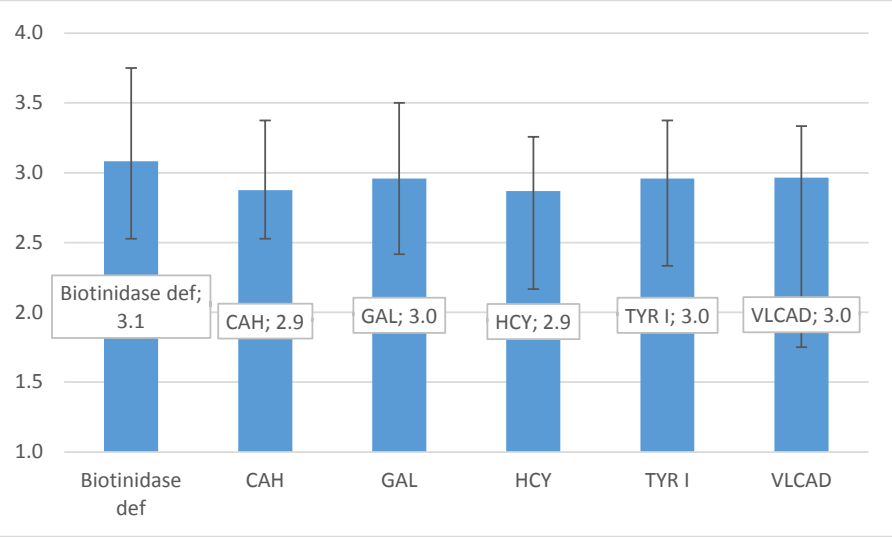
The most striking finding of this MCDA pilot testing is that, in the base case analysis, the composite scores show little differences across the six diseases, therefore providing a low discriminative power to select priority diseases (Figure 4). The median composite scores range from 2.9 to 3.1 with a large range of individual composite score by evaluator.

As indicated in the methodology section we also explore five additional methods to compare composite scores per disease, for instance by using common scores for all evaluators or by using means (data in report). Figure 5 shows that the ranking of each disease is somewhat dependent upon the method chosen to calculate the composite scores. However, we notice some interesting patterns, since biotinidase deficiency is ranked first or second in all methods, GAL is first or second in four of the six methods while HCY and VLCAD is systematically ranked low. (because of ex aequo's in the scores the total number by rank is not always six).

Potential reasons for these rather small differences in the global composite scores of these diseases have been discussed in the steering group:

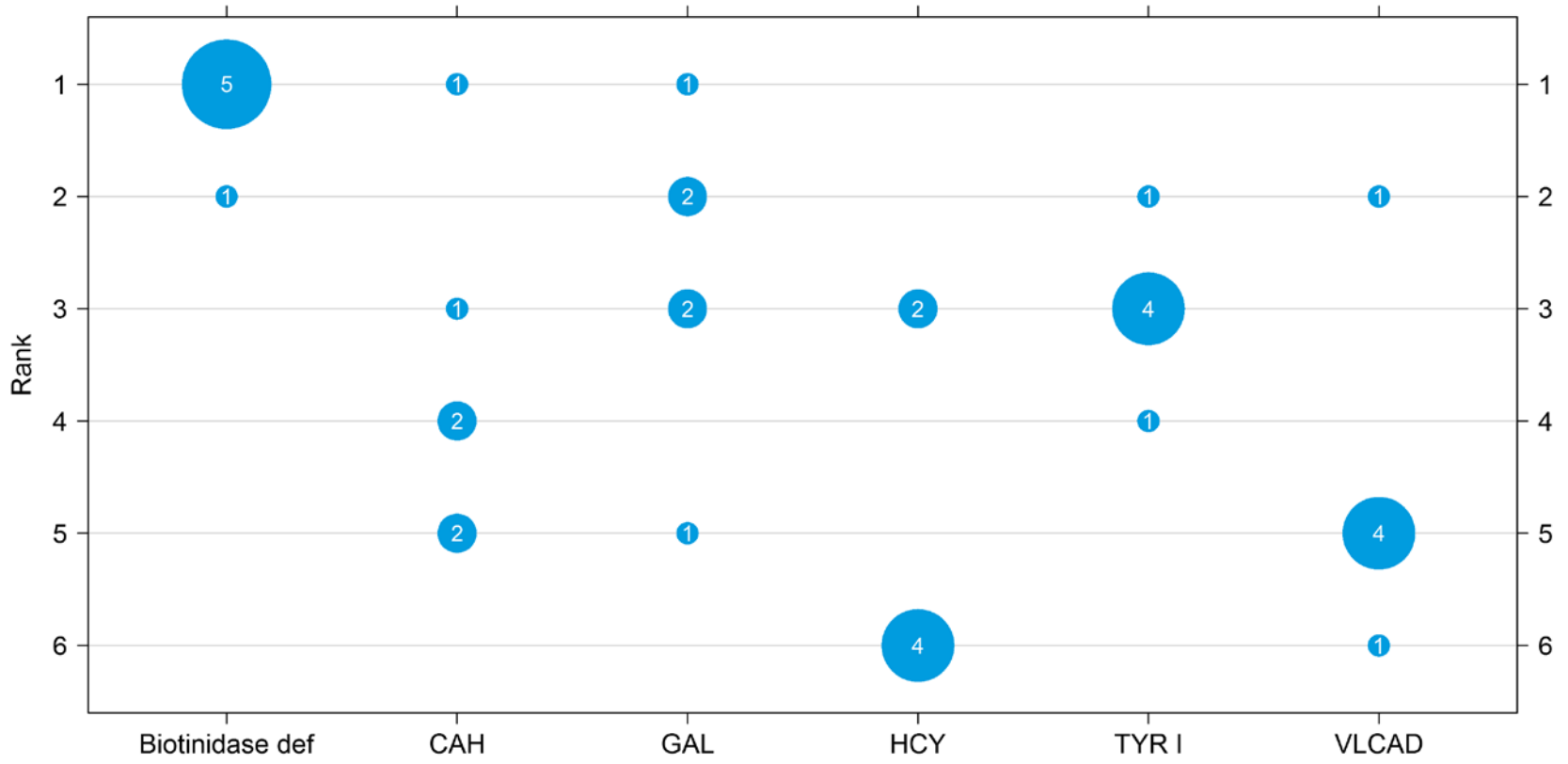
- The six diseases selected for this pilot testing have probably a similar importance and interest for NBS screening in Belgium because they were already selected in one community and thus were previously considered as being worthwhile to be screened.
- The narrow scale for scores, i.e. from 1 to 4, may tend to decrease the potential differences in individual scores, and a scale from 1 to 10 might provide different results.
- The indicators used are the median or mean ("central tendency" measures), which hide the differences between the individual scores.

Figure 4 – Base case analysis: composite scores by disease (+ range of individual composite scores over all evaluators)



Biotinidase def.: Biotinidase deficiency; CAH: Congenital Hypothyroidia; GAL: Galactosaemia; HCY: Homocystinuria; TYR I: Tyrosinemia Type I; VLCAD: Very long-chain acyl-CoA dehydrogenase

Figure 5 – Ranking by disease for the six methods used to calculate composite scores



CAH: Congenital Hypothyroidia; GAL: Galactosaemia; HCY: Homocystinuria; TYR I: Tyrosinemia Type I; VLCAD: Very long-chain acyl-CoA dehydrogenase

8. CONCLUSIONS

In this study, we test an MCDA method to rank diseases that can be detected through neonatal blood screening by priority. Building a consensus about the selection and uniform definitions of the criteria proved to be a more complex issue than anticipated, and required a considerable amount of time and exchanges. In this study, total composite scores show little differences across diseases and thus provide a low discriminative power to rank diseases for NBS.

The ranking of diseases changed somewhat according to the method used to calculate the composite scores, making further discussion about this method necessary.

Several members of the steering group considered it inappropriate to base policy and decision-making on such complex issue only on a composite score by disease. However, because this weighing and scoring induces more reflection and prompts to make arguments explicit, results of this exercise were felt useful to feed the decision-making.

This method could be improved by developing a more detailed definition of each criterion, splitting up criteria that may have antagonist components, considering a larger scale to weigh and score, and by paying careful attention to the balance of the profiles of those participating in the evaluation.

Future exercises to rank diseases should preferably include a wider range of diseases and can benefit from the lessons learned in this pilot test.

9. KEY MESSAGES AND LESSONS LEARNED FOR THE FUTURE

- Decision making about including an (additional) disease in a neonatal blood screening programme is a complex matter with many dimensions. For those decision making processes formal decision-making methods are increasingly used.
- Inspired by the experience of the INESSS agency in Québec we conducted a pilot study to test the usability of a *Multi-Criteria Decision Analysis (MCDA)*. Together with a steering group with representatives and experts of the communities in Belgium and stakeholders we decided to test this instrument on the six diseases that are currently only screened for in one of the two communities.
- The selection of the decision criteria is not easy. The heterogeneous weighs given for some criteria shows that this process should preferably run through multiple cycles to specify the precise content of the criteria and, if necessary, to add content or to split criteria with the aim to avoid misunderstandings.
- Evaluators can have diverging points-of-view. Attributing a weigh and score for each of the criteria calls for a broad discussion between evaluators.
- Scoring diseases for each of those criteria makes it unavoidable to collect information for each disease and for each of those criteria; This information should include evidence on epidemiology, available tests and interventions, including the organisational, health economical and ethical aspects. This information should be made available to all evaluators so they can all judge with the same basic information.
- To determine the weights for each criterion and for the scoring of the diseases for each of those criteria a LIKERT scale with only four points may be too limited and not enough discriminating. We could not really answer this question. The six diseases were, for decision making, probably in the same zone between acceptable or non-acceptable for a screening programme, in the first place since they had already been selected by one community.

- During the composition of the panel of evaluators careful attention should be paid to make sure that they represent all relative viewpoints and to avoid that essential arguments are overlooked.
- During the steering board meetings it was mentioned that it might be useful to consider an ‘exclusion score’ to indicated if a necessary condition for screening for a specific disease is not fulfilled.
- The exact way to calculate global composite scores slightly influences the global composite score and the ranking of diseases. Again, with the six selected diseases we could not really answer this question as the global composite scores were too close.
- It is not the aim to determine decision making on inclusion or exclusion of specific disease in a screening programme exclusively on a MCDA ranking. The aim of this exercise is to allow discussions about this decision making to be more objective and transparent. This way, a better cohesion between successive discussions can be reached.

RECOMMENDATIONS[c]

To the competent decision makers

- **Internationally there is no consensus on diseases for which early Neonatal Blood Screening through a general screening programme is necessary. There is also no consensus on how to organise decision making on this. For those complex question a Multi-Criteria Decision Analysis method (MCDA) can provide an additional instrument in decision making.**
- **MCDA should not be considered as a pure mathematical decision making process but it can help to structure decision making and make it more transparent. The systematic use of such a method can also lead to increased consistency in decision making.**
- **The criteria used need to be discussed in depth before the weighing and scoring takes place and precisely defined by the full group of evaluators. Probably some of the criteria will need to be disentangled when they are related both to the seriousness of disease as to the possible treatment, since they could have opposing effects on the score.**
- **Further research is needed for an optimal aggregate measure of the composite score of an MCDA.**
- **For every disease, the necessary information should, by criterion, be assembled to allow evaluators to score with the same basis knowledge about the disease.**
- **For composing the group of evaluators attention should be given to a reasonable balance between the different parties involved (decision makers, medical and laboratory specialists, patient representatives, ethicists, etc.) to ensure all opinions and preferences are well represented.**
- **Information on the costs and benefits of the diagnostic trajectory after a positive screening test (including false positives) and the ultimate treatment of the true positives is difficult to find. We recommend to organised and systematic and long-term follow-up of all positively screened neonates to allow for better information in the future.**

[c] Only KCE is responsible for the recommendations

COLOPHON

Title:	Multi criteria decision analysis to select priority diseases for Newborn blood screening - Synthesis
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