

EXPERT QUESTIONNAIRE ON [HEALTH CONDITION]: PRIMARY PREVENTION STRATEGIES, DIAGNOSTIC TIMING, TREATMENT AVAILABILITY AND EFFECTIVENESS, AND RESEARCH GAPS.

1 INTRODUCTION

This questionnaire is part of the [NEED project](#), led by the Belgian Health Care Knowledge Centre (KCE) and Sciensano. The project aims to measure health-related unmet needs of patients and wider society across a variety of health conditions. It seeks to highlight the most significant unmet needs in order to steer research, improve healthcare, and develop new interventions where they are most needed. The results are made available in an open-access [database](#).

We are contacting you as an expert in [health condition], to provide information on five indicators related to the unmet needs associated with [health condition]. Your responses are highly valuable and will be treated anonymously. If you have any questions please contact [email of the expert in charge of the data collection]. [mailto:](#)

Privacy Policy

1. Data confidentiality

Your data will be processed in compliance with the Belgian Law of 30 July 2018 on the protection of natural persons in relation to the processing of personal data, and Regulation (EU) 2016/679 of 27 April 2016, which entered into force on 25 May 2018, on the protection of natural persons with regard to the processing of personal data and on the free movement of such data.

You have the right to request the [name of the centre responsible for the study] to access, rectify, erase and transfer your personal data and to restrict and object to the processing of your data. To do so, please contact the [name of the centre responsible for the study] as the Data Controller responsible for the processing of your personal data by e-mail [e-mail address] or by letter to be sent to [address of the centre responsible for the study].

Please do not hesitate to contact us with any questions or comments you may have about the processing of your data. If you wish, the [name of the centre responsible for the study] Data Protection Officer is available to provide you with further details regarding the protection of your personal data. S/He can be contacted by e-mail [e-mail address].

If this proves to be insufficient, you are free to lodge a complaint about the way your data are being treated with the [nationality] supervisory authority responsible for enforcement of the data protection legislation: Data Protection Authority (DPA) at [authority postal address] or by e-mail [authority e-mail address].

2. Data retention period

The primary data is kept on record for two years after the reports are published. The aggregate (anonymous) data is retained in the archives of the NEED database indefinitely but only visible until a newer estimate becomes available.

3. Processing purposes

The objective of this survey is to collect data on the unmet needs associated with a specific health condition, in terms of primary prevention, diagnostic timing, treatment effectiveness and availability, and research gaps. The results will appear in the NEED database, with the aim of providing evidence to inform policy-making and steer research and innovation into the areas with highest unmet needs.

In addition, the results might also figure in publications (e.g. reports, journal articles) dedicated to the health condition.

4. Consent statement

- I hereby affirm that I have been informed in writing, in a way that is understandable to me, of the nature, method and purpose of this study.
- I hereby accept and agree to take part in this scientific research.
- I understand that I am free to withdraw at any time until the data are recorded and stored in the database without having to state any grounds for doing so and without this having any kind of consequences for me.
- I declare that I have no major conflict of interest that could compromise the accuracy or objectivity of the information provided, and I confirm that I have submitted the separate Conflict of Interest Statement truthfully and to the best of my knowledge.

I understand that my data is collected and recorded on a confidential basis, and that the Principal Investigator guarantees their confidentiality.

To continue please first accept our survey privacy policy.

- ☐ I accept the survey privacy policy

2 GENERAL BACKGROUND

G1: Please indicate which country you will provide information for as an expert in [health condition]: *(Mandatory)(List)*

G2: Please indicate your clinical specialty: *(Mandatory)(Longtext)*

G3: Have you conducted academic or research work related to [health condition] (e.g., teaching, scientific publications, clinical trials, literature reviews, or participation in guideline development)? *(Mandatory)(SC)*

- ☐ No
☐ Yes

G4: How many years of clinical, academic, or research experience do you have in the area of [health condition]? *(Mandatory)(SC)*

- ☐ 0 - 5 years
☐ 5 - 10 years
☐ 10 - 20 years
☐ > 20 years

G5: Please select the population affected by [health condition] you will be reporting on in this questionnaire: *(Mandatory)(SC)*

- ☐ Paediatric (0-17 years)
☐ Adult (18+ years)

2.1 Primary prevention in [country]

By **primary prevention**, we consider measures that aim to prevent a disease from ever occurring. Thus, its target population is healthy individuals. Primary prevention commonly institutes activities that limit risk exposure or increase immunity to prevent (sub)clinical disease from developing in a susceptible individual.^a For example, immunisations, tobacco-control policies, and pre-conceptual folic acid supplementation are forms of primary prevention.

PR1: To what extent are effective primary prevention strategies currently implemented in [country] for [the health condition]? *(Mandatory)(SC)*

Please mark a cross next to the category that applies:

^a Kisling LA, Das JM. Prevention Strategies. [Updated 2023 Aug 1]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2025 Jan-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK537222/> (last access: 25 August 2025)

IMPLEMENTATION OF EFFECTIVE PRIMARY PREVENTION STRATEGIES	DESCRIPTION	RESPONSE
Not at all implemented	No primary prevention strategies are implemented, even though the health condition is (partly) preventable using well-known approaches.	
Minimally implemented	Few or isolated efforts exist to address the health condition through primary prevention strategies, with little impact on population health.	
Moderately implemented	Efforts are underway to prevent the health condition through established primary prevention strategies, but gaps remain in coverage, resources, or effectiveness.	
Fully implemented	Comprehensive and fully resourced primary prevention strategies are in place, reaching a wide population with demonstrated effectiveness in preventing the health condition.	
Not applicable	The health condition cannot currently be prevented through primary prevention strategies.	
I don't know		

PR2: Please provide reasons for your choice, along with any additional information you deem relevant (e.g. references to key studies): *(Mandatory) (Longtext)*

2.2 Timing of diagnosis of [disease] in [country]

This section aims to assess whether there are any unmet needs in terms of **delayed diagnosis** of [disease] or in terms of **over-diagnosis** of [disease].

Important note:

In some diseases, there may be no clearly defined point in time at which diagnosis consistently improves outcomes such as survival, disease progression, complication rates, or quality of life. Decisions regarding the initiation of treatment or management are, in these cases, more influenced by symptom severity, patient preferences, and the broader psychosocial context.

Examples of diseases that could fit into this category: the common cold, irritable bowel syndrome, Alzheimer's disease.

If you consider this to be the case, please select "Not relevant" in questions DIA1 and DIA4.

2.2.1 Delayed diagnosis

By **delayed diagnosis**, we refer to a situation where a disease is identified later than would have been optimal. This refers to cases where an earlier diagnosis could have enabled more effective treatment or intervention, improving outcomes such as quality of life or survival.

DIA1: How common is **delayed diagnosis** of [disease] in [country]? (*Mandatory*) (SC)

- ☐ Rare (e.g. <10%)
- ☐ Occasional (e.g. 10-20%)
- ☐ Moderate (e.g. 20-30%)
- ☐ Frequent (e.g. 30-40%)
- ☐ Very frequent (e.g. >40%)
- ☐ Not relevant: see explanation above
- ☐ I don't know

DIA2: If you consider that delayed diagnosis of [disease] in [country] is **moderate, frequent, or very frequent**, please indicate the main reasons (check all that apply): (*Mandatory*)(SC) (*Condition: DIA1=moderate or frequent or very frequent*)

- ☐ The diagnostic tools in use are insufficiently effective
- ☐ There is no screening
- ☐ There is screening but it is limited to certain populations or limited in uptake
- ☐ The disease typically presents with late symptoms
- ☐ There is lacking awareness among healthcare professionals
- ☐ There is lacking awareness among the general public
- ☐ Stigma limits early presentation
- ☐ Other reason(s): _____

DIA3: Please provide reasons for your choice, along with any additional information you deem relevant (e.g. references to key studies): (*Optional*)(Long text)

2.2.2 Over-diagnosis

By **over-diagnosis**, we refer to the diagnosis of a disease at a stage when it is not (yet) causing harm—such as pain, dysfunction, or suffering—and for which early treatment offers no advantage over treating it later. In these cases, early diagnosis may lead to unnecessary treatment, including side effects, or anxiety-inducing watchful waiting.

DIA4: To what extent is **over-diagnosis** of [disease] a concern in [country]? (*Mandatory*) (SC)

- ☐ No concern
- ☐ Slight concern
- ☐ Moderate concern
- ☐ Considerable concern
- ☐ Major concern

- ☐ Not relevant: see explanation above
- ☐ I don't know

DIA5 If you consider that over-diagnosis of [disease] in [country] is a **moderate, considerable, or a major concern**, please indicate the main reasons (check all that apply): *(Mandatory) (MC) (if DIA4=moderate or considerable or major concern)*

- ☐ High disease awareness among the general public
- ☐ High disease awareness among healthcare professionals
- ☐ Existing screening programme
- ☐ Available over-the-counter and online diagnostic tests
- ☐ The disease typically presents with early symptoms
- ☐ Other reason(s): _____

DIA6: Please provide reasons for your choice, along with any additional information you deem relevant (e.g. references to key studies): *(Optional)(LongText)*

2.3 Treatment

By treatment, we mean the use of **therapies** that are based on scientific evidence and that have been prescribed, recommended, or that are carried out by a healthcare professional with the goal of **promoting healing, relieving symptoms, preventing complications, repairing injury, or restoring health**.

Treatment may include various interventions such as **medication, surgery, medical procedures** (e.g., joint aspiration), **medical devices** (e.g. pacemaker), **physical therapy, psychological therapy**, and **other forms of (clinical) interventions** (e.g., hydration, artificially provided nourishment) to cure the specific disease or condition, to target its symptoms, to modify its course, or to prevent complications.

2.3.1 Treatment effectiveness in [country]

TT1: In [country], to what extent do you consider that the current treatment/management of [health condition] is effective in the patient population? *(Mandatory)(SC)*

Please **evaluate the overall effectiveness in real-world clinical practice for the majority of the [health condition] patient population**. If for instance, a treatment exists which is highly effective but only for a minority patient subpopulation (e.g. patients with a specific genetic mutation), then downgrade the overall treatment effectiveness score to take this into account. Please rest your evaluation on (high-quality) scientific evidence as much as possible.

Please mark a cross next to the treatment effectiveness category that applies:

TREATMENT EFFECTIVENESS	DESCRIPTION	RESPONSE
No treatment exists	For this disease, there is currently no recognised treatment	
Not effective	With current treatment, the disease has a poor prognosis or the symptom burden remains very high	
Somewhat effective	The current treatment is not curative or disease-modifying and important symptoms/impact on quality of life persist	
Moderately effective	With current treatment, the disease has a good prognosis over time (e.g. disease-modifying), but some symptoms or complications persist	
Very effective	The current treatment is curative or disease-modifying and patients can achieve a very good quality of life without complications over time	
Lacking evidence	There is lacking scientific evidence to make an overall evaluation of treatment effectiveness	
I don't know		

TT2: Please provide reasons for your choice, along with any additional information you deem relevant (e.g. references to key studies): (Mandatory)(LongText)

TT3: In the context of [health condition], can you identify any significant **treatment effectiveness gaps** (i.e. treatment 'not effective' or only 'somewhat effective') for specific patient subpopulation(s)? (Mandatory)(SC)

*Example 1: In the context of **cystic fibrosis**, the treatment can be considered 'not effective' for patients with **nonsense mutation on both alleles** (5% of the patient population).*

*Example 2: In the context of **tuberculosis**, the treatment can be considered 'not effective' for patients with **multi drug-resistant TB** (8% of the patient population).*

- ☐ Yes, I identify 1 subgroup
- ☐ Yes, I identify 2 subgroups
- ☐ Yes, I identify 3 subgroups
- ☐ Yes, I identify 4 subgroups
- ☐ Yes, I identify 5 subgroups
- ☐ No
- ☐ I don't know

3.1. Concerning the first subgroup (condition TT3=yes I identify 1 subgroup, yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

TTGAP1: Which subgroup of [disease] patients experiences a significant gap in treatment effectiveness?

Example 1: In the context of cystic fibrosis, this could be "patients with nonsense mutation on both alleles". **Example 2:** In the context of tuberculosis, this could be "patients with multi drug-resistant TB".

TTGAP2: Subgroup prevalence if known (% of the patient population): (Optional)(Numerical Input) (condition TT3=yes I identify 1 subgroup, yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

TTGAP3: How effective do you consider the treatment in this subgroup to be? (Mandatory)(SC) (condition TT3=yes I identify 1 subgroup, yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

- ☐ Not effective
- ☐ Somewhat effective

TTGAP3BIS : Please provide reasons for your choice(s), along with any additional information you deem relevant (e.g. references to key studies): (Optional)(LongText) (condition TT3=yes I identify 1 subgroup, yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

3.2. Concerning the second subgroup (condition TT3=yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

TTGAP4: Which subgroup of [disease] patients experiences a significant gap in treatment effectiveness ? (Mandatory)(LongText)(condition TT3=yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

TTGAP5: Subgroup prevalence if known (% of the patient population): (Optional)(Numerical Input) (condition TT3=yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

TTGAP6: How effective do you consider the treatment in this subgroup to be? (Mandatory)(SC) (condition TT3=yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

- ☐ Not effective
- ☐ Somewhat effective

TTGAP6bis : Please provide reasons for your choice(s), along with any additional information you deem relevant (e.g. references to key studies): (Optional)(LongText) (condition TT3=yes I identify 2 subgroups, yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

3.3. Concerning the third subgroup (condition TT3= yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

TTGAP7: Which subgroup of [disease] patients experiences a significant gap in treatment effectiveness ? (Mandatory) (Longtext) (condition TT3=yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)

TTGAP8: Subgroup prevalence if known (% of the patient population): *(Optional)(Numerical Input) (condition TT3=yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)*

TTGAP9: How effective do you consider the treatment in this subgroup to be? *(Mandatory)(SC) (condition TT3=yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)*

- ☐ Not effective
☐ Somewhat effective

TTGAP9bis : Please provide reasons for your choice(s), along with any additional information you deem relevant (e.g. references to key studies): *(Optional)(LongText) (condition TT3=yes I identify 3 subgroups, Yes I identify 4 subgroups, Yes I identify 5 subgroups)*

3.4. Concerning the fourth subgroup *(condition TT3= Yes I identify 4 subgroups, Yes I identify 5 subgroups)*

TTGAP10: Which subgroup of [disease] patients experiences a significant gap in treatment effectiveness ? *(Longtext) (Mandatory)(condition TT3=Yes I identify 4 subgroups, Yes I identify 5 subgroups)*

TTGAP11: Subgroup prevalence if known (% of the patient population): *(Optional)(Numerical Input) (condition TT3=Yes I identify 4 subgroups, Yes I identify 5 subgroups)*

TTGAP12: How effective do you consider the treatment in this subgroup to be? *(Mandatory)(SC) (condition TT3=Yes I identify 4 subgroups, Yes I identify 5 subgroups)*

- ☐ Not effective
☐ Somewhat effective

TTGAP12bis : Please provide reasons for your choice(s), along with any additional information you deem relevant (e.g. references to key studies): *(Optional)(LongText) (condition TT3=Yes I identify 4 subgroups, Yes I identify 5 subgroups)*

3.5. Concerning the fifth subgroup (condition TT3= Yes I identify 5 subgroups)

TTGAP13: Which subgroup of [disease] patients experiences a significant gap in treatment effectiveness ? (Mandatory)(LongText)(condition TT3= Yes I identify 5 subgroups)

TTGAP14: Subgroup prevalence if known (% of the patient population): (Optional)(Free text) (condition TT3=Yes I identify 5 subgroups)

TTGAP15: How effective do you consider the treatment in this subgroup to be? (Mandatory)(SC) (condition TT3= Yes I identify 5 subgroups)

- ☐ Not effective
- ☐ Somewhat effective

TTGAP15bis : Please provide reasons for your choice(s), along with any additional information you deem relevant (e.g. references to key studies): (Optional)(LongText) (condition TT3=Yes I identify 5 subgroups)

2.3.2 Treatment availability in [country]

TT5: To what extent do you consider that the current treatment/management of [health condition] is available in [country]?(Mandatory)(SC)

By **availability** of treatment, we mean **the logistical and financial accessibility** to the patient population.

Availability problems might concern key treatments (whether used on- or off-label) that are:

- not authorised,
- authorised but not commercialised,
- commercialised but not reimbursed,
- subject to regular stock problems,
- treatments that are not available due to a lack of specialised professionals or resources to perform or provide the treatment (e.g. physiotherapy, psychological therapy, surgery)

Please mark a cross next to the treatment availability category that applies:

TREATMENT AVAILABILITY	DESCRIPTION	RESPONSE
No treatment exists	For this disease there is currently no treatment anywhere	
Not available	Treatment(s) for the disease exist but none are available in [country] (e.g. not authorised or not commercialised)	
Important availability problems	At least one key treatment for the disease is authorised and commercialised in [country], but	

	other existing key treatments are not, or treatment is commercialised but very expensive/not reimbursed, or insufficiently available for other reasons (e.g. important lack of specialised professionals)	
Some availability problems	Treatment is available for a majority of the population but too expensive for some, or there are some stock problems or some other availability problems (e.g. some lack of specialised professionals)	
No availability problems	Treatment is available for all patients without particular obstacles	
I don't know		

TT6: Please provide reasons for your choice, along with any additional information you deem relevant (e.g. references to key studies): *(Optional)(LongText)*

2.4 Research gaps

RG1: To what extent do research gaps related to [health condition] currently exist in the following areas: (Mandatory)(SC)

For each disease aspect, please mark a cross next to the level of research gap that applies.

	No gap: Comprehensive knowledge exists; no significant research gaps are identified in this area.	Minor gaps: Some gaps in knowledge exist, but they are not critical barriers to understanding and effective action in this area.	Moderate gaps: Significant knowledge gaps exist that limit understanding or effective action in this area.	Major gaps: Critical gaps in knowledge severely hinder understanding or effective action, and require urgent research attention	I don't know	Not applicable
PATHOPHYSIOLOGY						
Knowledge of the causative factors, pathophysiological mechanisms & natural history						
DIAGNOSIS						
Knowledge of diagnostic criteria, disease classification, & effective diagnostic tools						
PHARMACOPREDICTIVE BIOMARKERS						
Knowledge on methods to predict disease sensitivity to treatment						
PROGNOSTIC BIOMARKERS						
Knowledge on methods to predict disease progression						
TREATMENT						

Knowledge on effective treatments, including preclinical research & high-quality clinical trials						
PRIMARY PREVENTION						
Knowledge on effective primary preventive strategies						
INEQUITIES						
Research gaps in specific patient populations (e.g. defined by ethnicity, socioeconomic status or other)						
OTHER						
e.g. optimal therapeutic pathways, patients' experiences of treatment, compliance, etc.						

RG2: Please specify which “other” aspect(s) have a research gap:*(Mandatory) (LongText) (if RG1-other=minor or moderate or major)*

RG3: If you have identified moderate or major research gaps, please describe what specific knowledge these gaps concern. *(Mandatory)(Longtext) (condition : pathophysiology=moderate OR major; Diagnosis=moderate OR major; pharmacopredictive biomarkers=moderate OR major; prognostic biomarkers=moderate OR major;treatment=moderate OR major; primary prevention=moderate OR major; inequities=moderate OR major)*

RG4: Please provide reasons for your choices, along with any additional information you deem relevant (e.g. references to key studies):*(Optional)(LongText)*

COLOPHON

Title: TOOL 6: Expert questionnaire – Supplement

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Disclaimer:

- This report describes a toolbox for NEED studies, based on the NEED team's experiences. As the tools and instruments will be adapted regularly, based on new insights, experiences and knowledge, the user should make sure to use the latest versions.
- The external reviewers commented on a (preliminary) version of the report. They did not co-author the report and do not necessarily agree with its content.
- This report does not contain policy recommendations that require approval by the Board of KCE. It has been reviewed by Belgian experts, but has not followed the formal validation procedure by independent academic experts. The report will likely be updated regularly based on progressive insights.
- Only the KCE is responsible for errors or omissions that could persist.

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