High-level Conference

Health-related needs as drivers for healthcare policy and innovation

Brussels, April 17 & 18th 2024 – Report

KCE
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Belgian Health Care Knowledge Centre

NIHDI

famhp
federal agency for medicines and health products

Introduction

Important amounts of public money are invested to ensure that patient needs are addressed. This includes funding of research into innovative healthcare interventions; incentives for (pharmaceutical) companies to develop products addressing unmet needs -through direct financing, subsidy or regulatory mechanisms, public procurement and reimbursement

However, decisions on the authorisation and reimbursement of health care interventions are often supply-driven, i.e. following a request of a developer, rather than based on previously defined priorities drawing on evidence about patient and societal needs.

To ensure that governmental expenditure and public funding are used to address the real needs of patients or society and that limited resources are allocated in the most efficient way, needs should be defined independently of the interventions/products that are in the pipeline. It is therefore important to know where needs are highest. Unmet patient needs may relate e.g. to increased life expectancy or quality of life. Societal needs may relate e.g. to the need to reduce healthcare-related costs or the need for affordable preventive treatments for (contagious) conditions.

The EU has a crucial role in these mechanisms through its important research funding programs, as well as through awarding regulatory incentives.

A life-cycle approach is needed to create a needs-driven system, involving (1) the identification of the highest unmet needs, both on the individual and on the societal level, (2) the creation of smart and predictable incentives towards health technology developers to steer R&D activities towards the highest needs and (3) approval and reimbursement processes that take performance of new products on these needs into account. This requires a strong collaboration at the EU level.

The defined priority needs should guide decisions on the granting of various public incentives and enable prioritisation in the allocation of resources. They furthermore assist HTA bodies and payers in making assessments and appraisals of whether new treatments respond to the real needs of patients and society.

Against this background this Belgian Presidency Conference aims to:

- Propose a common methodology to identify and assess unmet needs and define priorities in a transparent and evidence-based way.
- Call the Commission to draft an EU strategic plan to adequately and effectively respond to the identified priority patient and societal needs, taking into account all types of health intervention, and aiming at the coordination of all kinds of public support and incentives.

Its ultimate goal is to trigger an evolution from a supply-driven towards a more needs-driven healthcare policy, both at the EU and Member State level.

Speakers and attendees include representatives from Member States, the EU institutions and other international organisations, stakeholders, and academia.

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Session Summaries

Opening session

Welcome Address and Introduction by Frank Vandenbroucke

Frank Vandenbroucke (Deputy Prime Minister and Minister of Social Affairs and Public Health, Belgium) welcomed participants by emphasising the need for health authorities to find and finance solutions to health problems. He highlighted the importance of researchers providing creative solutions and pointed out that health authorities usually come into play at the end of the development chain to decide on benefit package coverage. He questioned whether these decisions address the most critical needs of patients and the population, advocating for steering innovation upstream to direct research toward pressing needs. Vandenbroucke introduced the conference theme, stressing the first step of identifying needs. He mentioned KCE's methodology, which provides clarity to developers on funding priorities, and underscored the importance of the Union's role in research and development (R&D) financing and incentives. It is all about a paradigm shift, how can we create an evidence database to identify, and use it to set priorities, as well as better orient our incentives. He expressed the need to cover how we can integrate these policies at national level. He also pointed out that addressing patient needs encompasses a wide range of solutions, from prevention and mental health care to high-tech devices and pharmaceuticals, emphasising that needs should be defined independently of the products.

Patient Testimony on Unmet Needs Related to Kidney Disease

Annemie Heselmans shared her journey as a dialysis patient, having lost both her kidneys to a resistant infection. Dialysis, the cornerstone of her life, necessitates three weekly sessions, making it difficult to maintain a normal life. She highlighted disparities in access to home dialysis across the EU and called for better options, noting the lack of technological advancements in dialysis over the past decades. Heselmans questioned the urgency of innovation in this area and described feeling like a prisoner to her condition. She advocated for the development of dialysis technology, artificial kidneys, and transplantation, emphasizing the EU's role in supporting member states to improve and organize practices that address the shortages of organ transplants. She urged the audience to work together to push for more investments in solutions that ensure all patients have access to the care they need, highlighting the burden of dialysis on patients, healthcare systems, and costs, which will only increase with the rising number of patients.

Session 1: Assessing disease-specific health-related unmet needs: creating the evidence base

Ann Van den Bruel (General Director, KCE) opened the session by emphasizing the importance of documenting patient and societal needs to drive innovation and the necessity to broaden the scope beyond pharmaceuticals. She highlighted that KCE has focused on unmet medical

needs in its research since more than a decade and referenced a milestone report of 2016 on the appraisal of unmet medical needs in the context of early access schemes. **Ann Van den Bruel** also reiterated the necessity for independent, solid, and reliable unmet need studies.

Irina Cleemput (Scientific Programme Director at KCE and lead of the NEED project) set the scene, explaining that despite major advances and innovations in healthcare, major unmet needs remain: for some diseases, there is no effective treatment yet, services are inadequate or unaffordable, and there are still neglected diseases on which little research is done. Moreover, evolutions in healthcare, and economic and social changes, have led to a changing society, with new challenges and new unmet needs: antimicrobial resistance, mental health problems, environmental pollution and pressure on the healthcare budgets. The current market mechanisms are not providing a solution. There is a disbalance in the pipeline of pharmaceutical trials, with certain healthcare domains being highly underserved. This leads to the exclusion of many patient populations from innovation. Moreover, there is a limited commercial interest beyond drug discovery.

Because pharma companies are accountable to their shareholders, they have to identify the investments with the highest expected return on investment. This steers the R&D pipeline and, as a consequence, the decision agenda of regulators and pricing and reimbursement agencies. This makes the current pharmaceutical innovation, development and approval approach highly supply-driven. Even though the initial investment decisions of companies will depend on the chances of success in the later stages of the development lifecycle, there is little steering by the authorities at the beginning of this lifecycle. Investments are targeted to areas where there is a demand or demand can be created, but demand is not necessarily equal to priority unmet needs.

Authorities have to rely on what the developer provides as information regarding the unmet needs their product is addressing. However, this evidence is increasingly becoming weaker. There is a trend towards accelerated marketing approvals, based on lower levels of evidence and no requirements for proof of added therapeutic value compared to the standard of care in terms of patient-relevant outcomes. German researchers assessed 216 drugs that entered the German market between 2011 and 2017. For 58% of these drugs, no proof was provided of added benefit over standard of care in terms of patient-relevant outcomes. Yet, they received marketing authorisation as they were deemed to do more good than harm, and several EU member states are paying for them.

Several scientific papers show that fast approvals based on, for example, single-arm trials, non-randomised trials, and surrogate endpoints do not only lead to uncertainty regarding the clinical value but also harm patients and society. And yet, despite this lack of evidence of added benefit in 40 - 60% of drugs, a recent study found that companies recoup their investments in 3 to 4 years, a little longer in case of conditional marketing authorisation.

Another important point is that the market-driven approach and focus on pharmaceutical solutions to health problems ignores the potential of other solutions. An example of a publicly funded clinical trial was given, investigating two management strategies for irritable bowel syndrome in primary care: a drug therapy (standard of care) and a smartphone FODMAP-lowering diet application. Already after 4 weeks, a significantly higher response rate was observed for the diet, which persisted during follow-up. Response was associated with significant improvement in quality of life, anxiety, depression and somatization, compared to

baseline. Many studies demonstrate that other solutions to unmet needs, like prevention, nonmedical interventions, social care etc should not be ignored. Also, the development and implementation path should be guided by the identified highest patient and societal needs, for these other types of solutions.

Therefore, we should aim at a system where unmet patient and societal needs are identified upfront, so that they can be used to define priority needs for research, innovation and policy, which can then be communicated to the research community, including research funders, who steer research to these domains with high priority needs.

Charline Maertens de Noordhout (Expert in health services research at KCE) presented the NEED framework, which was introduced as a structured approach designed to identify unmet health-related needs.

The objective of the NEED project is to create a framework with criteria for identifying patient and societal unmet needs to inform and support the development of more needs-driven healthcare policy and innovation. The project also assessed the applicability of the NEED approach to rare diseases and applied the framework to two selected health conditions: melanoma and Crohn's disease. The ultimate goal of the NEED project is to create a sustainable framework to develop, host and maintain an evidence database on patient and societal unmet needs in different health conditions.

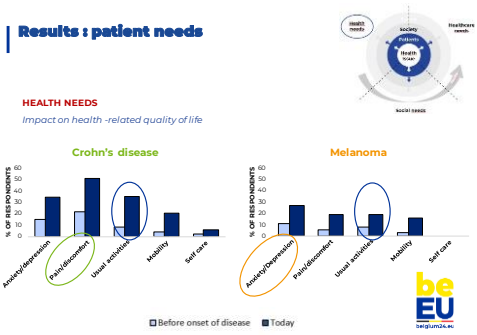
To achieve the objectives of the NEED project, a specific NEED approach has been developed, which includes a 4-step implementation model, consisting of (1) identifying health conditions with potential high unmet needs, (2) prioritizing these conditions, (3) collecting evidence, and (4) disseminating the results. Calls for topics (health conditions) for unmet needs research at both Belgian and European levels were launched to identify health conditions with potential high unmet needs, resulting in 352 submissions. Many submissions asked for more unmet needs research about long COVID.

The NEED assessment framework was developed through literature reviews and consultations with stakeholders and experts. It categorizes needs into health-related needs, healthcare needs, and social needs, considering patient, societal, and future perspectives, with a strong emphasis on equity. The framework encompasses 23 needs criteria and 43 indicators to evaluate whether these needs are being met. A concise description of the framework is available via the following link: EU presidency high-level conference: brochure (health-needs.eu)

For the patient dimension, 13 criteria are defined, covering health, healthcare, and social needs. The societal dimension includes 8 criteria, while the future dimension has 2 criteria. Equity is a transversal dimension, ensuring the fair distribution of unmet needs among different population groups. A Delphi survey at the EU level is currently underway to validate the NEED assessment framework, with results expected to be published by January 2025.

Claudia Schönborn (Clinical Expert at KCE) presented example results from the two case studies carried out to test the NEED framework. These case studies focused on Crohn's disease, a chronic inflammatory gastrointestinal disease, and melanoma, the most aggressive form of skin cancer. For both diseases, information was collected on the unmet needs from both patient and broader societal perspectives using various data sources: a patient survey, interviews, expert input, scientific literature, and public health databases.

The gathered data highlighted areas with unmet needs within each disease and allowed for comparisons across diseases, which can be useful for prioritization in decision-making processes. Diagnostic delays were one of the key examples of significant unmet needs: in the case of melanoma, this hinged on the prolonged time patients sometimes took before deciding to consult a doctor after the onset of the first symptoms, while in the case of Crohn's, it concerned the clinical delay in diagnosis once initial contact with a healthcare professional was made.



Example of results

Other unmet needs included the lack of treatment effectiveness, particularly for advanced stages and rarer types of melanoma. For Crohn's disease, challenges included insufficient symptom control, reliance on a trial-and-error approach, and loss of response over time.

In addition, unmet needs in terms of inequity were highlighted, with lower socioeconomic status in both populations associated with excess morbidity and mortality

Given that the NEED project is still in its early stages, future steps were discussed, such as the necessary adaptation of the framework to paediatric and acute diseases, the establishment of the NEED database, and collaboration across EU countries.

Isabelle Huys (Professor and head of Unit Pharmacology and Pharmacotherapy at the Faculty of Pharmaceutical Sciences of KU Leuven) presented the results of the applicability assessment of the NEED approach to rare diseases. Extensive research was carried out to evaluate the applicability of the NEED framework, taking into consideration the unique challenges faced by individuals with rare conditions and the broader societal implications of these diseases. The researchers concentrated both on the inherent characteristics of rare diseases (e.g. heterogeneity and complexity of diseases) as well as on the methodological issues related to measuring unmet needs in the limited patient population for each rare condition.

The NEED framework was overall found to be applicable to rare diseases and minor suggestions for modifications were made. To characterize patients' unmet health-related needs, some additional criteria were identified. These criteria relate to the often lengthy and tortuous diagnostic pathways, including the number of misdiagnoses or the number of medical visits needed before a final diagnosis.

Strong and active engagement with key stakeholders, including patients, caregivers, rare disease associations, academia, healthcare providers, researchers, regulators, and industry partners, as well as European reference networks, is essential to ensure the success of a need assessment in rare diseases.

Irina Cleemput closed the presentations on NEED, explaining that The NEED approach can support decision making processes regarding policy priorities and scientific innovations, it can help to reduce suboptimal allocation of scarce healthcare resources and by this increase population's health and well-being. Reference was made to several reports of the WHO and of the Panel for the Future of Science and Technology for the European Parliament, STOA, that also highlighted the need to transform the current system.

Finally, some recommendations were formulated: to ensure reliability of the evidence included in the database, the database should be set up and maintained by independent researchers that coordinate research into unmet needs and ensure standardisation of procedures and methods for unmet needs research, provide advice, training and support to researchers in collecting data on unmet needs.

International collaboration with established initiatives such as the European health information portal and in the future the European Health Data Space will be key. For the governance, we can get inspired by existing infrastructure and initiatives, like the European Centre for Disease Control, Joint Research Centres, the International Horizon Scanning Initiative (IHSI) and the Beneluxa Initiative. Liaison with decisionmakers will be crucial to make sure the work performed by the infrastructure is relevant for decision-making processes. Without collaboration and coordination at the EU level, fragmentation of initiatives will remain and markets will not be reshaped to being patient-centred, and needs-oriented.

Jasper Claessen (Coordinating Policy Advisor, Department of Pharmaceutical Affairs and Medical Technology at the Dutch Ministry of Health) focused on the role of pharmaceutical developments in the discussion on unmet medical needs. He explained that the Netherlands started in 2019 with a reflection on whether society is clear about what medicines it needs and what it is willing to pay for them.

Acknowledging that medicine development has increasingly become more complex, with many different actors involved and many financial transactions taking place along the drug development path, the need emerged to first better understand the financial ecosystem that drives the development of novel medicines and the outputs it generates, before further reflections about needs-driven policy measures could start.

Key actors in the medicine development process include public actors (universities, hospitals, public funding bodies), companies (start-ups, biotech, big pharma and contract research organisations) as well as investors (angel investors, venture capital, mergers and acquisitions).

Public funding is important for early discovery, but private investments drive drug development in later stages, and here, the expected financial return on investment ultimately determines whether a product is further developed up to launch. In the estimation of the expected financial return, the global governments and (private) insurance companies' expected willingness to pay for new drugs obviously influences the development of novel drugs and the distribution across therapeutic areas.

Medicine development is expensive and successes have to compensate for all the failures in the ecosystem and the costs of capital. However, as ultimately public health budgets reward investment and pay for the new products, it is important to make sure that the public payer is paying for what he actually needs. Hence, it should be defined which medicines society needs most and how much it is willing to pay for the medicines it needs. This requires a societal debate. The NEED project can be a helpful tool for that.

Jasper Claessen concluded by saying that it is important to continue to invest public resources in basic research, especially in areas of unmet medical needs (e.g. novel antibiotics, and therapy-resistant depression). Moreover, he recommended the development of a toolbox with legal terms for universities to make licensing contracts to guarantee public interest when licensing public intellectual property companies and openly asks the question of whether public medicine development can be advanced to a higher value point (e.g. clinical

development) to better safeguard public interests after IP licensing.

On the EU level, he emphasized the importance of collaboration to collectively determine areas of unmet medical needs, explore ways to coordinate public research funding in those areas, and of creating a level playing field by introducing a European framework to guarantee public interest in licensing deals on public intellectual property.

Roisin Adams (Head of Strategy and External Engagement at the National Centre for Pharmacoeconomics and chair of the HTA Coordination Group) explained how unmet needs have been arising within Health Technology Assessment (HTA). Willingness to pay has been a major topic in HTA. Through cost-effectiveness frameworks, public payers are expressing their willingness to pay for clinical benefits.

In the EU HTA Regulation (HTAR), unmet needs, or, in this case, Unmet Medical Needs (UMN), are mentioned several times:

1. Article 7 – UMN as a criteria for choosing medical devices to be assessed.
2. Article 17 – UMN as a criteria for selecting products for joint scientific consultation
3. Article 22 – It may be used as a criterion for identifying emerging health technologies and potentially doing an assessment early.
4. Voluntary cooperation under the HTAR – should take into account "impact on patients, public health and healthcare systems"

However, even though addressing UMN is one of the clear motivations of the HTA Regulation, no definition is provided within the Regulation.

The joint clinical assessments (JCA) performed at the EU level in the context of the EU HTA Regulation will feed into the national procedure, where the discussion of willingness to pay will start. The willingness to pay can be different across member states. JCAs are syntheses on products that will be launched.

Besides JCAs, joint scientific consultations (JSC) will be performed in the context of the EU collaboration on HTA. JSCs involve discussions with companies on what their plans are. In this context, it is possible to get into the detail of whether the evidence that is going to be provided incorporates the outcomes that will address the unmet needs.

Finally, the EU HTA Regulation also requires work on Emerging Health Technologies (EHT), where the objective is to identify products that are coming our way and assessing whether they are likely to address unmet patient or societal needs. In this context, Roisin highlights that it is possibly even more important to identify what is *not* coming our way, i.e. which needs will remain unmet in the years to come.

In Ireland, clinical need is included in the legislation, as a criterion to decide to include something in the pharma benefit package. It is a criterion products have to address and it is assessed on a product level. Probably, it should be looked at on a more general level, because opportunity costs should also be considered. If unmet needs are not balanced against cost-effectiveness, there will be huge opportunity costs. Horizon scanning is key, because it will allow us to assess whether what is coming is addressing our needs, *and* identify areas with high unmet needs where nothing is coming. This should allow us to steer the debate on where investments are needed.

Ann Van den Bruel closed the session, referring to the popularity of the concept of patient-

centredness in policy documents, while concrete actions often remain relatively modest. The NEED framework puts patients really at the center and might be a concrete step towards patient-centered innovation and policy. Collaboration will be key, not only between stakeholders such as researchers, patient organizations, regulatory bodies and pricing and reimbursement agencies, but also between EU member states. A combination of push and pull policies to obtain the innovations patients and society really need will be necessary.

Session 2: Steering innovation: smart research funding and regulatory incentives

This session sought to clarify why some promising developments reach the market while others do not, despite their potential to address unmet needs. It analyzed the financial ecosystem of pharmaceutical R&D to understand what drives private investments, focusing on bottlenecks for a needs-driven approach and discussing how to address these. The discussion revolved around the critical stages of development where public incentives could make a difference, steering research and development funding towards defined needs, and determining the forms public incentives should take beyond the willingness to pay.

Hugues Malonne (CEO, Federal Agency for Medicines and Healthcare Products), opened the session by highlighting the need for a strategy to steer R&D towards the most pressing needs. He emphasized the importance of evidence-based identification of needs followed by smart and coordinated allocation of incentives to provide predictability, greater return on investment, and healthcare interventions tailored to patient needs.

Saskia van der Erf (Managing Partner, SiRM) presented two studies commissioned by Dutch health authorities. The first study, about the financial ecosystem of pharmaceutical R&D, was commissioned by the Dutch Ministry of Health to get a better understanding of the ecosystem and how it operates. The Ministry felt the need for this study to enable well-informed debates. A consortium consisting of Strategies in Regulated Markets (SiRM), L.E.K. Consulting LLP (L.E.K.) and RAND Europe conducted the study.

This study's overarching conclusion is that a drug's expected financial return ultimately determines whether it is developed up to launch. Assessment of expected financial return incorporates multiple interconnected factors, including but not limited to commercial potential, investment cost, availability of capital, the potential for scientific and medical advancement, strategic fit and risk. The relative importance of these factors varies by investor and evolves as drugs move through the drug-development continuum.

A drug's expected financial return is driven by its expected revenue potential. Therefore, global governments and (private) insurance companies' expected willingness to pay for new drugs considerably influences the supply of novel drugs and the distribution across therapeutic areas. Other important factors influencing the supply of novel drugs are the pace and nature of scientific advances, the ability of R&D systems to leverage data and digital technology advances to inform innovative clinical trial designs, and regulatory developments.

The second study, also commissioned by the Dutch Ministry of Health concerned the output of the current ecosystem. The objective of the study was to assess for which conditions the current ecosystem produces many or few new drugs. In order to explore this, SiRM analysed the EMA brand name registrations for 33 conditions with the highest annual total burden of disease. Different

patterns of development were observed :

- A quarter of the conditions have seen continuous drug development since 1995.
- Thirty percent of the conditions show a more erratic pattern: a clear increase in the last decade preceded by limited development.
- Fifteen percent have seen little or no development in the past decade after a period of greater development.
- For about thirty percent of the conditions there has been little to no drug development. This can be caused by various reasons, such as the lack of clear pharmacological targets or the existence of other (non-pharmacological) treatment options.

The panel discussion featured Anton Muyldermans (Advisor at the office of Thomas Dermine, State Secretary for Recovery, Strategic Investments and Science Policy); Karin Sipido (former chair of the European Commission's Scientific Panel for Health and current member of the Open Research Europe Board), Emer Cooke (Executive Director of the European Medicines Agency) Els Torreele (Policy Associate at the UCL Institute for Innovation & Public Purpose), and Nathalie Moll (Director-General of EFPIA).

Anton Muyldermans discussed the drivers and bottlenecks of private investment, noting the importance of development cost, patient population, and expected willingness to pay. He called for coordination and inclusion of patient perspectives to address potential market failures. **Karin Sipido** provided examples from the area of cardiovascular diseases, where financial and regulatory bottlenecks hinder drug development, emphasizing the need for coordination and strategic public investments. **Emer Cooke** discussed the regulatory role in supporting SMEs by providing better scientific advice and improving predictability to de-risk the process.

Els Torreele criticized the financialization of the system, noting that an alleged 60% of new drugs have no demonstrated added therapeutic value at the moment of marketing authorization, and called for a public sector-driven approach to address unmet needs. She emphasized the need for public leadership with a vision and mission to foster an end-to-end ecosystem based on health needs and deliver medical benefits. **Nathalie Moll** highlighted the importance of a partnership approach and removing barriers, de-risking investments, and ensuring regulatory predictability and infrastructure. She questioned who defines unmet needs and called for collaboration to improve the ecosystem.

Emer Cooke cautioned against a narrative that labels a substantial proportion of new medicines as lacking added therapeutic value. Feedback from patients and doctors indicates that a wider choice of new therapeutic options helps to better meet different patient needs and optimise treatment recommendations post-authorisation. **Anton Muyldermans** stressed the importance of investing in need identification and coordination of public funding. **Nathalie Moll** and **Els Torreele** emphasized the need for equitable access and structuring research financing to deliver on patient needs. **Emer Cooke** called for alignment and collaboration across stakeholders, and **Karin Sipido** agreed, noting the importance of strategic prioritization and a concerted stakeholder consultation in this process.

Hugues Malonne closed the session by recalling that back in 2010, the Belgian Presidency already put innovation and solidarity high on the agenda through a conference on innovation and solidarity followed by Council conclusions urging among others for prioritisation of research investments based on criteria such as the debilitating impact of the disease on the patient and expected added value for patient and society. Those conclusions are still valid today. We can and we must make better use of public money to meet public health needs.

Irene Norstedt (Director – People: Health and Society, Directorate, DG Research & Innovation, European Commission) concluded the day by emphasizing the importance of evidence-based, needs-driven healthcare policy and the power of collaboration to address unmet needs, particularly in rare diseases, personalized medicine, and antimicrobial resistance. The session underscored the need for strategic use of public healthcare spending and collaboration to define and address unmet health needs effectively.

Session 3: A needs-driven approach is a holistic approach

The session focused on raising awareness that pharmaceutical products are only one of many potential solutions to unmet health needs. It aimed to identify the position of pharmaceutical products within a holistic approach and demonstrated how a needs evidence database can be used to find diverse solutions for health-related needs. The panel, made up of patient representatives, physicians, pharmacists and policy advisors, discussed what the biggest challenges are in moving towards a holistic approach to addressing unmet medical needs, how research and development in the field of non-pharmaceutical therapies can benefit from the creation of an unmet needs list and how to ensure the support of all stakeholders.

The session started with a testimony of **Maxime Fastrez** (Associated Director, Gynaecology-Obstetrics department, University Hospital Brussels) who described the story of a patient with endometriosis who could not be helped with pharmaceuticals. He stressed that individual patient needs demand individual approaches especially where pharmaceuticals cannot be used.

Pedro Facon (Deputy CEO, Belgian Institute for Health and Disability Insurance) opened the scene by thanking the KCE team for highlighting the difference between unmet medical needs and health-related needs and stressed that a needs-driven system is not the same as a demand-driven system. Even though the current system provides innovations that do respond to healthcare-related needs, necessary therapies are still missing. Discussion about percentages of low-value innovation will not help individual patients but each percent of improvement matters. Public leadership and partnership, collaboration and alignment are necessary to shape the market so that solutions can be found, not only for pharmaceutical and medical devices but also for the organisation of healthcare systems and care pathways.

Marc Dooms (Professor Emeritus of Hospital Pharmacy at KU Leuven) noted that regulations on orphan devices are only present in Japan and the US, with other countries lagging behind. Currently, many devices are frequently used off-label. He pointed out that surgery is entering a new phase with custom-made devices containing active ingredients, highlighting the urgency for regulatory measures. **Marc Dooms** proposed offering incentives to promote the development of more orphan devices, suggesting that engaging in discussions with the medtech industry could lead to improved incentives.

Anca Toma (Executive Director, European Patient's Forum) called for inviting, enabling, empowering, and recognizing the contributions of patients. She praised the clever KCE approach for bringing forward the unquantifiable aspects of patient needs through the NEED project, acknowledging its limitations while emphasizing its significance as a crucial step. **Anca Toma** stressed the importance of addressing unmet needs beyond healthcare, including the

psychosocial impacts. She concluded by stating that addressing unmet needs should be the first criterion in innovation, focusing on equity, rights, values, and co-creation. She reiterated the importance of early engagement with patients, listening to their needs, and embedding this dialogue in the development of solutions, often finding that patients prefer management strategies over medication.

Ole Johan Bakke (Vice-President, Standing Committee of European Doctors - CPME) emphasized the unequal access issues, particularly affecting vulnerable groups such as drug users and those living in rural/deprived areas. He highlighted the necessity for a new approach that focuses on understanding what patients and populations truly need, rather than solely on what the healthcare system can provide. **Ole Bakke** stressed the critical importance of the doctor-patient relationship and sufficient time allocation, which in turn require clear national priorities and adequate financing. He underscored the significance of funding and prioritization, especially for groups experiencing the most significant unmet needs. Additionally, **Ole Bakke** emphasized the need for governments and the individual to take responsibility ensuring that unintended use of the health care system is reduced, rather than relying solely on external financing.

Ece Özcelik (Health Policy Analyst, OECD Health Division, Directorate for Employment, Labour and Social Affairs) advocated for a paradigm shift towards a needs-based approach that adapts interventions from one region to another, incorporating behavioural change components. She acknowledged the challenge of getting everyone on board in a time of misinformation and disinformation, emphasizing the need for clear communication on opportunities and challenges and measuring buy-in over time with indicators. **Ece Özcelik** stressed the importance of cost-effectiveness analysis, particularly with public budgets under constraint, urging investment in areas with the highest return on investment for society. She emphasized that a holistic approach may look different for each country but should prioritize effectiveness and adaptability. Overall, she emphasized the need for a new way of thinking that integrates the needs framework and identifies gaps to prioritize future actions.

Claudia Marinetti (Director, Mental Health Europe**)** stressed that a holistic approach is essential due to the increasing burden of disease and current inadequacies in mental health care. She criticizes the over-reliance on pharmaceuticals and the inappropriate application of physical diagnostic models to mental health, emphasizing that these approaches are not recovery-oriented. **Claudia Marinetti** advocates for considering intersectionality and involving people in defining needs, promoting a co-construction approach rather than mere consultation. She believes in empowering patients and valuing their expertise to avoid making assumptions. **Claudia Marinetti** emphasizes that any framework for mental health must involve people from the beginning, using a co-creation approach. She underscores the importance of caregivers in mental health care. She argues that co-creation increases trust in the health system and promotes buy-in, focusing on hope and relationships as crucial elements.

In conclusion, **Pedro Facon** stated that the approach to healthcare innovation should emphasize patient co-creation, where solutions are developed in collaboration with patients to meet their specific needs. Solutions should be adapted to local or regional contexts and include buy-in of health professionals to ensure they are effective. It's important to continually evaluate the effectiveness of these solutions and innovate to address evolving needs.

Regulatory barriers and inequalities can pose challenges to implementing solutions. Efforts to

address these barriers, such as advocacy and lobbying, are essential. Equally important is ensuring that all solutions, including those facing challenges like orphan medical devices, have the opportunity to enter the market and be adopted.

Decision-making processes should be structured to carefully evaluate solutions, taking into account regulatory hurdles and inequalities, to ensure fair access and effective implementation. This comprehensive approach is vital for fostering a healthcare environment where all patients can benefit from innovative solutions tailored to their needs.

Session 4: Towards needs-driven health care systems

Session 4 of the conference focused on transitioning health systems to be more needs-driven. The session addressed three key topics: steps to develop needs-driven health systems, collaborative efforts at the European level, and the potential impact on the R&D ecosystem.

Jo De Cock (former CEO, Belgian Institute for Health and Disability Insurance) introduced the session by emphasizing the need for health systems to evolve from passive beneficiaries to active drivers of innovation. Historically, decisions were often ad-hoc and not always transparent, leading to suboptimal budget use. He called for the establishment of dynamic change management processes and highlighted that health systems won't remain blind payers. They will anticipate and address issues like mental health proactively. Despite progress in several areas, such as evidence-based medicine, health technology assessment (HTA), and health system performance assessment, the NEED project demonstrates that more can be done. It aims to improve evidence-informed health policy decision-making and optimize resource allocation by focusing on identifying priority health, healthcare, and social needs from a patient and societal perspective, while taking equity into account.

The panel discussion, featuring Florian Schmidt (Deputy Head of Unit, Medicines: Policy, Authorisation and Monitoring, DG SANTE, European Commission), Yannis Natsis (Director, ESIP), Ancel·la Santos (Senior Health Policy Officer, BEUC), Kim Helleberg Madsen (Head of Division, Danish Medicines Agency), and Jorge Mestre-Ferrandiz (Profesor Asociado, Universidad Carlos III de Madrid) delved into nuanced perspectives on needs-driven healthcare.

Florian Schmidt expressed the need to make specific progress, which is highlighted in the pharmaceutical strategy. The Commission proposes the use of incentives to encourage developers to focus on unmet needs areas in its legal proposal for the reform of the EU pharmaceutical legislation. He underscored the importance of maintaining simplicity in the system and guiding developers with clear evidence requirements.

Yannis Natsis emphasized the importance of providing clarity and consistency and the need to find an implementable and explainable definition of unmet need. He linked this to incentives and to affordability, stressing that approved medicines should be accessible to patients. He stressed the significance of evidence-based healthcare. He also mentioned the need to strengthen the dialogue with EMA and emphasized the need for robust, complete and

qualitative data at the time of pricing and reimbursement. He stressed not to undermine the HTA regulation and urged to rationalize pharmaceutical expenditure. He urged a shift in the prevailing paradigm where the industry categorizes everything as an unmet medical need, emphasizing the need for buyers to organize themselves and align evidence requirements.

Ancel·la Santos drew attention to the realities faced by patients due to treatment unavailability and pricing barriers. She called for increased public sector stewardship and interventions across the lifecycle of a medicine, including regulatory support for nonprofits, reevaluation of drug pricing models and early entry of generics. She advocated for increased upfront funding with attached conditions, citing potential cost savings. She emphasized the importance of fair pricing to avoid overpaying and sending the wrong signals, particularly in light of the excessive prices of new treatments. She proposed joint procurement as a means to enhance access beyond crises.

Kim Helleberg Madsen underscored the significance of a shared understanding of (highest) unmet needs for informed decision-making. He emphasized the link between unmet need and HTA. HTA bodies will likely play a role in moving towards a needs driven system and when selecting products for joint assessments, unmet need will be one of the first criteria. He emphasized the necessity of establishing a comprehensive view of the pharmaceutical pipeline to prioritize effectively, underlining the importance of horizon scanning. He stated unmet needs will play an important role in terms of prioritizing.

Jorge Mestre-Ferrandiz highlighted the enduring challenge of affordability amid advancements in new medicines. He underscored the significant time lag (15 years) between the implementation of certain decisions and their tangible effects on access for patients. He advocated for evidence-based policies and robust stakeholder engagement to navigate the evolving landscape of needs-driven health care, with a need to improve the trust among all stakeholders. He also called for breaking silos in healthcare delivery and emphasized the multifaceted nature of health outcomes. He raised concerns about perverse pricing systems and referred to international reference pricing. He advocated for global treaties allowing for pricing differentials across countries.

Jo De Cock concluded the session underscoring the need for a common understanding, for better communication to the broader public, for better understanding of how health systems function, and for strategic investment in health system management to prepare for future challenges and opportunities. The NEED project, providing a framework for needs-driven policies, aims to foster a more equitable and effective healthcare system.

Closing session – High-level debate

Jo De Cock opened the closing session, saying that not everything is set. Push and a pull incentives remain to discuss. But one thing is clear: this project matters. This way of thinking matters. It could power up and show up which need could be met to reduce the burden of disease, and create readiness within our health system. We need a common research infrastructure. We need a strategic plan.

The high-level debate featured Frank Vandenbroucke (Deputy Prime Minister and Minister of Social Affairs and Public Health, Belgium), Karla van Rooijen (Deputy Director-General, Ministry of Health, Welfare and Sport, the Netherlands), Katharina Reich (Director-General for Public Health and Health

Systems, Ministry of Social Affairs, Austria), Eva Vodnik (State Secretary, Ministry of Health, Slovenia) and Sandra Gallina (Director-General, DG SANTE, European Commission).

Frank Vandenbroucke stated that we are partly sailing blind and that we have to deal with scarce resources. We need to better understand underlying dynamics, how innovation functions, and how our market functions. We also need a mutual understanding and a comprehensive life cycle approach to innovation, from target selection, development, and access, to cost-effectiveness analysis and price and reimbursement decisions. Gearing the system towards the most pressing needs is complex and we have to cope with an existing ecosystem. This won't be easy as it involves complex ethical and conceptual issues, but it is badly needed. To validate the data from Belgium collected through the NEED project, a comparison with other countries' data to validate, check and test the assessment framework proposed by the NEED project would be useful. The incentives set in the pharma legislation and in scientific policies could be improved in terms of cost-effectiveness assessment and but evidence generation. He called for an independent research infrastructure and a political debate on using evidence on unmet needs for future political decisions. Upscaling the NEED project to the EU level and giving it a proper clear place in the Health Union perspective, not only for pharma R&D, is the way to go.

Karla van Rooijen confirmed that we need this system very much. Healthcare costs are rising, not our budgets. We need to be clear about what we are willing to pay and we need a framework allowing us to be critical, to allow brave choices and decisions and sometimes say no. She found it important to work together on negotiations, in a co-creation process, with patients and industry. She mentioned that in this regard, next to the EU cancer plan, the Beneluxa Initiative is a good start, although we also need to think ahead. She found it important that the EU takes its responsibility, not only as a facilitator. The EU can do more with funding and the possibility of bringing countries together. We are too small to do it on our own.

Katharina Reich stressed the importance of both incentivizing private investment in areas of less interesting unmet needs and of improving transparency, especially in biotech. We know little about decision-making in pharma, for example when it comes to net costs. We could also consider to improve risk-sharing and other incentives to create a willingness to invest where it wouldn't be the case otherwise. For this, we need a transparent and evidence-based approach, and she is strongly committed to support and find necessary to sustain this political commitment. Small countries need collaboration on incentives, and we should focus on tailoring incentives for both big and small countries. Of course, this push for change will also have to be supported by industry.

Eva Vodnik focused on the challenges of identifying real unmet health needs. People can be influenced. This is why we need more data and scientific proof of what the unmet needs are. She took the example of overweight: people might ask for medicinal treatment, but this is clearly not the best answer. She further mentioned the added value of European Reference Networks both for research, and for treating patients.

Sandra Gallina said that it is also a political choice to focus on rare diseases. She acknowledged that unmet needs will be perceived differently by each actor but recognized that it is high time to come out from the supply-driven mentality and from the emotions. Europe is relatively well-positioned to address rare diseases thanks to the European Reference Networks, the HTA framework, and thanks to the dialogue existing among payers.

In his closing address, **Mr. Frank Vandenbroucke** stated that R&D is crucial to improve our systems and shift their frontiers to cover what the current system fails to respond to. That means to shift the

system towards a more needs driven approach. We lack evidence on unmet needs, we lack coordination and we make a suboptimal use of incentive. Patient needs should be one of the driving factors. We have to state clearly where the needs are high, and make this known early in the process. The KCE can help but the trajectory toward a comprehensive database is still long and collaboration is needed. In any case, setting up a list of unmet needs does not take away the responsibility of policy makers to take decisions, to set priorities and to make choices where needed, but those decisions can be fed by evidence.

Colofon

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