

Let's discuss the future of Unmet Medical Needs (UMN) in EU Policies

Report from a multi-stakeholder workshop





A Roundtable on Unmet Medical Need (UMN) took place in Brussels on 5 October 2022. Convened by EFPIA, it brought together representatives from European patient organisations and European medical societies, to discuss the European Commission's current thinking on UMN in the context of the 2020 Pharmaceutical Strategy, and to exchange views on potential implications for patients and their communities. Participants also shared thoughts on some of the wider needs impacting on patients' lives, related to, but going beyond UMN per se.

It was agreed that the meeting would be conducted in accordance with the Chatham House Rule. All participants have reviewed and approved this report as an accurate reflection of the discussion. See Annex I for the list of participating organisations.

Introduction, context and initial thoughts

In the framework of its 2020 Pharmaceutical Strategy¹, the European Commission is revising the basic pharmaceutical legislation, i.e., the 2001 Pharmaceutical Directive, the 2004 EMA Regulation, the 2000 Orphan Drug Regulation and the 2006 Paediatric Regulation. The strategy also marks a clear ambition to address UMN.

Addressing UMN is central to pharmaceutical innovation as the life science industry aims at developing treatment that will improve and extend patients' lives: UMN is a relevant concept throughout the lifecycle of medicines, from research prioritisation to drug discovery and pricing and reimbursement. Given the diversity of stakeholders and incentives involved across this lifecycle, how UMN is understood and applied can diverge significantly.

Despite these different perspectives, the broad concept of UMN has so far helped medicines to reach patients who need them the most. UMN is understood as a condition that is not adequately prevented, treated or diagnosed by authorised interventions². It is not a binary concept as the "need" is constantly evolving in line with availability of the latest science, technology, data, infrastructure and collaboration, and with the progression of diseases (chronicity vs. acute).

UMN depends on the patient perspective (e.g. the burden of disease on individual, quality-of-life, new formulations of existing treatments that are less intrusive in daily life), society (e.g. incremental improvements in disease management), and healthcare system perspective (e.g. resource allocation, the cost-effectiveness of treatments).

The Commission's stated objective for the revision of the pharmaceutical legislation is to better direct innovation towards areas of UMN while addressing availability, access and affordability, and fostering innovation. The Commission intends to establish a shared understanding of UMN through a definition and a set of criteria to be classified as meeting an UMN. This would be reflected in regulatory pathways to stimulate Research & Development (R&D) and innovation in those areas, and to frame intellectual property incentives and rewards.

At the time of the roundtable, the available information indicated that the Commission is planning to include a rather narrow definition of and criteria for UMN in the general pharmaceutical legislation.³

¹ https://health.ec.europa.eu/medicinal-products/pharmaceutical-strategy-europe_en

² [Unmet medical need \(efpia.eu\)](https://www.efpia.europa.eu/unmet-medical-need)

³ Politico Pro's publication of the Commission's Pharmaceutical Legislation Impact Assessment (29 Aug 2022). Access from: <https://pro.politico.eu/news/eu-pharma-reform-plan-faces-delay-after-red-light-from-commissions-scrutiny-board>

The Impact of a narrow definition

A narrow definition of UMN will lead to less R&D investment in the EU and to fewer treatments for patients (because of decreased certainty within the Intellectual Property framework). Many new potential treatments providing benefit for patients would not be included. Arguably, this would also have an implication at national level with the application of UMN in the context of Health Technology Assessment (HTA) and pricing and reimbursement decisions.

Another potential consequence of a narrow definition would be the undermining of solidarity across disease areas, creating possible “competition” with “second class diseases” and “second class patients” for whom their unmet needs are not recognised.

Europe’s research and development base is gradually eroding (48% of new treatments originate in the US, compared to 22% in Europe). Without a flexible and inclusive framework on UMN that can stimulate R&D, Europe cannot become a true world leader in medical innovation, one of the key objectives of the Pharmaceutical Strategy.

Similarly, the progress made in oncology over the past 20 years was not the result of one cure, but of a combination of different therapies. A narrow definition with a curative approach would make a similar combination of therapies impossible.

From a patients’ rights perspective, the definition of UMN should not be used to manage resources (health systems or otherwise). The understanding of UMN needs to cover the spectrum of prevention, early detection, quality of life, care, and cure.

Health inequalities also influence the perception of unmet medical needs where — due to the non-reimbursement of drugs and long waiting times in accessing innovative drugs — some doctors treat such medicines as if they simply did not exist.

“Were this narrow definition to have been in place for the past 20 years, the paradigm shift in the management of type 2 diabetes, and therapies that have had major benefits in the areas of neurogenerative diseases and CVD would simply not have been possible”.





< 6 mm

Uneven and blurred edge

Asymmetry

Color

Evolution

Patient and healthcare professionals' perspective on UMN in different disease areas

CANCER

There is a growing burden of cancer in Europe. While Europe provides some of the best cancer care in the world, there is an unmet need in terms of public delivery and support for cancer survivors. The upcoming regulations provide an opportunity to address this in terms of:



Taking a more comprehensive and holistic approach, encompassing the entire continuum of care or patient pathway: from prevention to diagnosis, treatment, and after-treatment quality of life



Including discussions on antimicrobial resistance (AMR) — recognising the need for new antibiotics. One cannot talk about cancer without talking about AMR



Recognising the need for innovation in terms of rare and ultra-rare diseases

If the definition of UMN is narrowed, the objectives of the European Health Union will not be reached and the objectives of Europe's Beating Cancer Plan (EBCP) and HTA regulation will be compromised.

The definition of UMN should be broad and inclusive otherwise it will deepen the disparities in cancer and set back what we have been building in cancer care for the past 20 years.

Having a narrow definition of UMN will have a major impact on disease areas such as lung cancer with all its subtypes. Patients identified through genomics have an improved prognosis with novel treatments.

In paediatric oncology, having a narrow definition would be counter-productive and would leave some patients behind. The definition should be multi-stakeholder and inclusive. UMN should be sufficiently flexible and include the quality of life of survivors; any fixed criteria would hamper innovation.

MULTIPLE SCLEROSIS

For MS patients, the first UMN is the aspect of progression. While there has been considerable research focusing on this, there are many needs that are not yet tackled appropriately, e.g., looking at the neurodegenerative process — the aim of delaying the onset of disability should be prioritised. Needs are highly varied so we cannot have a narrow definition that does not reflect this. It is important that the definition of UMN will improve the quality of life of a patient but be flexible enough to address those varied needs. A patient-centred approach is key, and patients should be involved in defining and applying the definition of UMN.

UNMET MEDICAL NEEDS – A PERSPECTIVE FROM THE MS COMMUNITY

Preventing progression, improving lives

Based on an interview with the European Multiple Sclerosis Platform (EMSP)

KEY POINTS

- Quality of life (not life-expectancy) should be the focus of disease management
- Lack of options is a common concern for people with MS
- Prevention, diagnostics, early intervention and halting progression are key
- Cognition, fatigue and the burden of treatment are often overlooked
- Patients must be included in conversations on research priorities, workplace relations and long-term care







DIABETES

In the diabetes area, the fundamental unmet medical needs are a cure for diabetes, and treatments to prevent or delay the onset of the disease, especially in the case of type 1 diabetes.

Addressing diabetes-related complications is important as well. There is a need for more effective treatment options to prevent or lower the risk for people living with diabetes of developing cardiovascular and kidney diseases, diabetes eye and foot diseases, neuropathy as well as other complications.

There is also a huge unmet need for medicines that help better manage glycaemia, so that people living with diabetes can achieve their therapeutic goals.

Personalised, adapted treatment and therapies for people living with diabetes are also of the utmost important, given the complexity of the disease.

UNMET MEDICAL NEEDS – A PERSPECTIVE FROM THE DIABETES COMMUNITY

Access to joined-up care and technology

Based on an interview with Bastian Hauck, CEO & Founder of #Dedoc

KEY POINTS

- Access to integrated care is not a top priority
- Stem cell therapies hold potential cure
- People with diabetes endure a significant burden in managing their condition
- Variation in access to technology depending on patient location and whether they have type 1 or type 2 diabetes
- Greater attention needed to infrastructure, urban planning and inclusion



CARDIOVASCULAR DISEASES (CVD)

There has been a major and worrying decline in industry investment in CVD. Out of 64 medicines in the PRIME scheme, which enables accelerated evaluation and patient access, only 2 are in CVD.

Conversations are taking place with the industry about why that has happened. One issue is that the regulators' interpretation of the UMN definition limits innovation from coming to the market by placing the focus on mortality. From the patients' perspective, innovation is also needed in promoting and/or preserving quality of life; these are also key endpoints.

UNMET MEDICAL NEEDS – A PERSPECTIVE FROM THE CVD COMMUNITY

Getting to the heart of what patients need

Based on an interview with Neil Johnson, Chief Executive of the Global Heart Hub

KEY POINTS

- Focus on disease management and quality of life rather than survival only
- Address the full impact of disease, including psychological and financial burden
- Innovation needed in heart failure, stroke, heart attack and heart valve disease
- Personalised medicines, genetics and regeneration therapy have a role to play
- Screening and early detection of familial hypercholesterolaemia is lacking
- A forum is needed where patients and stakeholders can discuss UMN





Open discussion

Participants were aligned that a broad definition of UMN is important: some participants reiterated that having a narrow definition might leave out some rare diseases. If the UMN definition is too narrow, it will also exclude tools for early detection and ignore the European Health Union's policy focus on prevention.

Only patients understand if/how much a therapy makes a difference in their lives — patient involvement must be embedded throughout medicines innovation and in related legislation. The concept of patient value is an important starting point to encourage innovation.

Defining UMN is not a “yes-no” issue — there are various elements at play and important nuances. The aim should be a consensus of understanding between patients' unmet needs and societal needs.

This requires input from a very diverse community of patients to arrive at a common understanding of the patient view of this concept and how we want it to be used.

Any EU definition or criteria must incorporate patients' views, be useful for targeting innovation (not too broad) but not too narrow and restrictive either.

The role of real-world data and evidence, and the European Health Data Space (EHDS), plays a critical role in innovation. We all want a broad definition, but priorities need to be made — these will evolve over time in different disease areas. Decisions must be evidence-based and future proofed.

The notion of excellence in science should be at the centre as the EU is lagging behind China and the US when it comes to research.



Wider considerations and unmet needs

Participants described some of the wider unmet needs faced by patients in managing their condition. Patients' needs reach beyond pharmacology. It is important to ensure that patients define the aspects that matter to them about living with their disease. For example, there are still significant disparities and unmet need in terms of public information about cancer, investing in prevention measures, accessing cancer care, delivering optimal treatment, and supporting cancer survivorship.

It is key that the upcoming regulation breaks down the existing silos and considers the whole patient pathway ensuring synergy with existing legislation such as the HTA Regulation, the European Health Data Space, and Europe's Beating Cancer Plan (EBCP). Genomics is hotly debated all around the world these days as it brings innovative therapeutic approaches. The EBCP clearly recognises the value of testing and targeted treatment, and personalised medicines.

Although genetic testing is crucial in identifying risk in a number of inherited cancers, some EU countries have no public access to biomarker testing. About 40% of cancers are preventable yet there is an unmet need in terms of education and investing in prevention care.

There are also several known policy and service delivery barriers: variations in workforce capacity and quality, reimbursement, data infrastructure and awareness of patients, the public and policymakers. These are all important factors which contribute to unequal access to therapies.

Another area that has not been focused on sufficiently is the impact of cognition and the mental well-being of patients. Evidence demonstrates that patients are facing physical, psychosocial, and emotional challenges to their well-being (e.g., fear of recurrence, living with uncertainty, adjusting to a new normal, and complicated co-morbidities) as they transition from treatment to the post-treatment phase.

When national policymakers talk about UMN, they mean health system or societal needs. Any discussion on UMN should consider the patient holistically. The definition of UMN should go beyond just looking at drugs and diseases. It must include other factors like socio-economic benefit and quality of life.





Conclusions

During the closing session, participants highlighted the high degree of alignment during the discussion, regardless of disease area.

In summary, the concept of UMN should avoid a narrow definition.

This implies that:



Patient values and goals should be the starting point for encouraging innovation



Patients should be involved meaningfully in the innovation, research, and regulatory process



UMN also need to cover the quality of life and treatment of patients

Having a broad understanding of the conditions associated with UMN was a point reiterated by several participants.

The suggestion of convening a multi-stakeholder forum as a possible way forward towards achieving a consensus on defining UMN was welcomed. There is the need to hear from others who may have different ideas on UMN — and to challenge each other constructively.

Investment in digital health, and real-world data in building patient relevant endpoints, was reiterated.

With more meaningful patient involvement, more evidence would be available to demonstrate the value to patients for regulatory evaluations. Better evidence will also be available for decision-makers involved in the pricing and reimbursement process, which in turn leads to improved access.

The need to imagine how the definition of UMN will be applied in years to come and the implications for future generations of patients was stressed.

It is time for the EC and policymakers to be ambitious in defining unmet medical needs together with stakeholders, reflecting their complexity and breadth.

The European Patients' Forum will be undertaking its own initiative to understand its members' perceptions on UMN and views on its use in policy decision-making. This will comprise desk research, stakeholder interviews, and an EPF membership meeting and survey, to inform a report and recommendations for policy and legislation.

Annex 1

Representatives from the following organisations participated in the roundtable on 5 October 2022:

Center for Innovation in Medicine (CIM)

Digestive Cancers Europe (DICE)

European Cancer Patient Coalition (ECPC)

European Cancer Organisation (ECO)

European Diabetes Forum (EUDF)

European Health Network (EHN)

European Hematology Association (EHA)

European Federation of Pharmaceutical Industries and Associations (EFPIA)

European Multiple Sclerosis Platform (EMSP)

European Patients Forum (EPF)

European Society of Cardiology (ESC)

European Society for Paediatric Oncology (SIOPE)

International Diabetes Federation Europe (IDF-E)