



EuropaBio Patient BioForum

Toward a Common
Understanding of Unmet
Medical Needs



1 DECEMBER 2022
EVENT REPORT

Foreword by MEP Pernille Weiss

“Addressing unmet medical needs represents an important challenge for Europe that will require positive tools to stimulate innovation and deliver for patients and society.

To meet that challenge, we need to mobilise the full strength of Europe’s scientific and academic excellence, the disruptive potential of SMEs, and the capacity of established companies to enable access to innovative medicines to support a resilient healthcare landscape.

Where innovation happens is as important as when it happens.

We need to support Europe’s rich life science sector with the right policies to encourage investments in disruptive science and technology that can deliver meaningful change and bolster our autonomy.

Addressing unmet medical needs will require us to break the silos and work together toward a common goal, making sure patients’ voices and experiences are heard. We need to adopt a balanced and careful approach and ensure no patients’ needs are unmet.”



Introduction

Bringing together multiple stakeholder groups, including patients and industry, the 2022 Patient BioForum was an opportunity to listen to patients' perspectives and experiences with unmet medical needs (UMN) and facilitate progress towards a common understanding on unmet medical needs. The discussions, held under Chatham House Rule, highlighted the subjectivity and the complexity of UMN for which a one-size-fits-all approach was not likely to succeed in improving the status quo. Importantly, the discussions revealed the need for further dialogue and collaboration between policy-makers and stakeholders, with patient voices duly represented, to help Europe tackle the challenges of UMN.

In recent years, UMN have been at the core of Europe's pharmaceutical and public health policies. Addressing UMN took centre stage in 2020 Pharmaceutical Strategy for Europe. One perceived shortcoming of current legislative frameworks is insufficiently stimulated development in some areas of 'UMN', such as rare diseases. The upcoming revision of the EU pharmaceutical legislation and orphan and paediatric legislation will aim at addressing UMN by stimulating and targeting innovation towards patients' needs.

A major hurdle to overcome is reaching a common understanding of UMN as stakeholders have different understanding of the concept based on different perceptions and expectations. UMN is a concept which can be used to identify patient, societal health needs, stimulate scientific progress and innovation towards meeting those needs. As science progresses, and with it the knowledge to address UMN, these needs will evolve over time from improving mortality, modifying disease progression, improving quality of life, burden of the disease, and potentially curing. In this regard, it is crucial that any future approach to all UMN is flexible enough to capture current and future needs of all patients as well those of society as a whole.

1 Putting patients at the centre

Participants to the Patient BioForum all agreed that patients' experiences with UMN should be duly considered when developing policy measures to tackle UMN. Important testimonials shared during the discussions brought to light the high burden experienced by many patients, even where treatments already exist. Patients are always seeking for better treatments or even a cure.

Putting patients at the centre means involving patients on an equal footing as other stakeholders and establishing systems that ensure patient voices are duly considered.

2 One size will not fit all

Discussions highlighted shared serious concerns about the unintended negative impact of a legal definition of UMN in the revisions of the general pharmaceutical legislation and orphan and paediatric regulations. Participants agreed that a legislative approach based on a rigid definition or criteria would not adequately reflect the complexity of UMN. A narrow approach to UMN runs the risk of discriminating against patients by creating a hierarchy of UMN and put at risk innovation and future treatments.

One size will not fit all means privileging non-legislative approaches to UMN that are scientifically sound, flexible, and future-proofed to accommodate the rapid evolution of treatments, science, and disease understanding.

3 A life-cycle and iterative approach

Participants noted that the revisions of the EU pharmaceutical legislation could have unintended consequences on patients' access to innovation and stressed the need to look beyond the revisions. Addressing UMN will require the coordination of various priorities to deliver better research, better science, better treatment, and better access for patients.

A life-cycle approach means considering UMN as a translation of basic science to innovative therapeutic development, but also encompassing care provision and patient access. This will require an iterative approach to accommodate for current and future UMN of people and society.

4 Investing in science and society

Discussions acknowledged that the lack of basic science or adequate scientific progress in some areas, such as rare or renal diseases, was inhibiting clinical development. Rapid advances in healthcare biotechnology and the potential of health data can accelerate the pace of scientific and technological progress. Investing in healthcare delivery is also to ensure patients can benefit from innovation.

Investing in science and society means supporting Europe's life science sector from scientific research to clinical development by positively rewarding innovation and maximising synergies between public and private actors through public-private collaborations (e.g. Innovative Health Initiative or Rare Disease Moonshot) to ensure life-changing treatments reach the most patients.

A call to action



At the close of the Patient BioForum, all participants endorsed the call to action for a multi-stakeholder forum to reach a common understanding of UMN.

“Calls on the Commission to convene a multi-stakeholder forum including patients, caregivers, regulators, healthcare professionals, industry, HTA bodies, and payers. This multi-stakeholder forum would support reaching a common understanding of UMN and, most importantly, how it may support scientific progress, translational research, and therapeutic development that will improve the lives of millions of patients.”

Do you support a call to action for a multi-stakeholder forum to conceptualise UMN?

Yes



No



Represented organisations:

- AbbVie
- Alliance for Regenerative Medicine
- BioMarin
- bioMérieux
- Children's Tumour Foundation Europe
- CSL Behring
- Eli Lilly
- EuropaBio
- European Confederation of Pharmaceutical Entrepreneurs
- European Federation of Pharmaceutical Industries and Associations
- European Patient Forum
- European Society for Paediatric Oncology
- European Society for Phenylketonuria and Allied Disorders Treated as Phenylketonuria
- EURORDIS
- Gilead
- Merck
- NHS European Office
- Novartis
- Pfizer
- PTC Therapeutics
- UK BioIndustry Association
- Voisin Life Science Consulting

About EuropaBio

EuropaBio, the European Association for Bioindustries, is the recognised voice of the European biotechnology sector, representing corporate and associate members, bio-regions and national biotechnology associations which, in turn, represent over 2300 biotechnology SMEs.

EuropaBio's Patient BioForum meets annually to facilitate the exchange of views and expertise on scientific, regulatory, and policy issues between patient organisations and the biopharmaceutical industry.

