



EuropaBio Patient Bioforum

Roundtable on Advancing Solutions
for Unmet Medical Needs

30 November 2023 – Event Report

Introduction

Bringing together representatives from the patient community, biotech community, and European Institutions, the 2023 Patient BioForum fostered exchanges on unmet medical needs (UMN), including expectations from the revision of the EU General Pharmaceutical Legislation (GPL) and beyond. The 2023 Patient BioForum was also an opportunity to issue multi-stakeholder recommendations to policymakers to ensure the GPL is fit for purpose to address patient needs and support innovation.

In recent years, unmet medical needs have been at the core of Europe's pharmaceutical and public health policies. To address UMN, the Commission proposed as part of the revision of the GPL to introduce definitions of UMN and of high UMN (HUMN) to promote patient-centric innovation.

The discussions, held under Chatham House Rule, acknowledged the shared objectives across stakeholders and the good intentions of the Commission's proposals on UMN but warned against unintended consequences. Participants highlighted the importance of broad and inclusive definitions within the legislation that can capture the complexity and impact of UMN on patients and carers while at the same time incentivise patient-centric innovation. The importance of involving patient representatives throughout the process, from drafting the definition to its implementation, was also agreed upon.

The discussions also revealed a shared understanding that the revision of GPL will not solve all issues and that Europe must leverage all available tools and connect the dots across entire ecosystems to build on incremental innovation toward breakthrough innovation for patients.

Importantly, the discussions revealed the value for continuous dialogue and collaboration between policy-makers and stakeholders, with patient voices duly represented, to help Europe tackle the challenges of UMN.

Broad and Inclusive Definitions

Building on the presentation of the proposals by the European Commission and the perspectives shared by representatives of the European Parliament, patient groups, and industry, participants agreed that the GPL must reflect patients' experiences living with UMN. Participants also expressed concerns about the definition of HUMN and warned that there should be no competition on who suffers the most.

This means that the definition in the legislation must be broad and inclusive and include elements such as quality of life and the benefit of incremental innovation on burden of disease and treatment. Additionally the development of scientific guidelines must include the voice of patients.

The Building Blocks of Breakthrough Innovation

Participants noted that the GPL must support innovation to pave the road to breakthrough innovation that can bring significant improvements to patients' lives. The development of innovative therapies is driven by the scientific understanding of a disease and its symptoms, progress in one disease area can help unlock science in others.

By continuing to support innovation, the GPL can help Europe leverage scientific and technological progress towards ever-better therapies and build toward a breakthrough.

Preparing for the Unknown Medical Needs

Participants agreed that the definition of UMN in the legislation will be in place in the coming decades and that it must be able to capture both current and future unmet needs. Developing therapeutic solutions to those currently unknown needs will require significant investment in science and innovation supported by a predictable incentive framework.

This means the definition of UMN must be broad enough to prepare for the unknown and take a life-cycle approach from the translation of basic science to innovative therapeutic development, but also encompassing care provision and patient access.

Focusing on Underserved Areas

Discussions revealed consensus on the need to use the GPL to do more for underserved areas, including rare diseases but also chronic and degenerative diseases that have a significant negative impact on patients and society.

Participants across stakeholder groups expressed support for an overarching European strategy for rare diseases and underlined the importance that the GPL supports innovation towards diseases currently without satisfactory treatments.

Focusing on Underserved Areas

Towards the end of the roundtable, participants all agreed that the GPL was no silver bullet, and that the EU must ensure coherence and complementarity across legislations and initiatives, including public-private partnerships (such as the Innovative Health Initiative), European programmes (such as the European Joint Programmes on Rare Diseases), or multi-stakeholder initiatives (such as the Rare Disease Moonshot).

Participants underlined the importance of the proper implementation of legislations such as the EU Health Technology Assessment Regulation or the European Health Data Space in facilitating development and access to innovative treatments. Discussions also noted the need to leverage non-health policy initiatives, such as the upcoming EU biotechnology and biomanufacturing initiative, to build infrastructure and capacity to manufacture and deliver next generation innovation to patients.

Represented Organisations

- Alexion Pharmaceuticals
- Alliance for Regenerative Medicines
- BioForum
- Biomérieux
- DIG PKU - Deutsche Interessengemeinschaft Phenylketonurie
- EFPIA - European Federation of Pharmaceutical Industries
- ELPA - European Liver Patients' Association
- EMSP - European Multiple Sclerosis Platform
- EPF - European Patient' Forum
- ESPKU - European Society for Phenylketonuria and Allied Disorders Treated as Phenylketonuria
- EUCOPE - European Confederation of Pharmaceutical Entrepreneurs
- EuropaBio
- European Commission - Directorate-General for Health and Food Safety
- European Parliament
- EURORDIS - Rare Diseases Europe
- FIPRA
- Gilead
- IPOPI - INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES
- Novartis
- Pfizer
- PTC Therapeutics
- RARE DISEASES GREECE
- Vertex Pharmaceuticals
- World Duchenne Organization

About EuropaBio

EuropaBio, the European Association for Bioindustries, promotes an innovative and dynamic European biotechnology industry. EuropaBio and its members are committed to the socially responsible use of biotechnology to improve quality of life, to prevent, diagnose, treat and cure diseases, to improve the quality and quantity of food and feedstuffs and to move towards a biobased and zero-waste economy.

About the Patient Bioforum

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 healthcare biotech industry.

Read more about our work at www.europabio.org.



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