



Press Release

European Health Forum Gastein 2023: European Rare Disease Research in crisis?

EHFG Session highlights urgency for greater patient involvement, cross-disciplinary collaboration, and funding for improving rare disease research outcomes.

Vienna / Gastein, 28. September 2023. This year's European Health Forum Gastein (EHFG) with its main theme "Health system in crisis – countering shockwaves and fatigue" has featured a session supported by AOP Health, which delved into the fascinating intersection of patient and health systems through the perspectives of academic and corporate researchers. It sparked a dynamic discussion about limitations, opportunities, and resources required for conducting ground-breaking research on rare diseases.

Innovation challenges or opportunities?

Rare disease research faces many challenges such as severely limited patient numbers, high costs and regulatory hurdles, impacting timelines and risk of failure. Peter Loeffelhardt, founder of the MPN advocacy network & patient representative, who moderated the panel, brought the research crisis to the point: "As a patient and patient representative, I urge for action in the face of the current rare disease research crisis, with limited progress in addressing unmet need. Let's unite, break barriers, and prioritize innovative solutions to transform lives. Patients can't wait – it's time to make a difference." Despite the fact, that the EU Orphan Drug Regulation aims to incentivise research for rare diseases, the panelists were quick to propose new ideas to make rare disease research more attractive.

Involve patients

Rare disease research needs patient collaboration to succeed: Their insights are crucial for research outcomes and should therefore be given a more active role in study design. The panelists agreed unanimously: research must aim for a positive impact on the patient – no matter if it concerns quality of life, minimizing side effects or developing therapies that meet patients' most urgent needs.

Susanne Greber-Platzer, head of the Department of Pediatrics and Adolescent Medicine, pointed out that disease related research and clinical studies bring hope to many patients with unknown diseases, but also a positive future perspective for targeted medications and interventions in chronic diseases. To improve the lives of children with inborn and complex diseases medical research can be seen as essential.



To make this happen, inter-disciplinary collaboration is key. According to Daria Julkowska, Scientific Coordinator of the European Joint Programme on Rare Diseases: "This crisis demands collaborative, cross-disciplinary action. Together, we'll redefine research approaches, leverage resources, and reignite hope for rare disease patients across Europe."

One best practice example "A European Partnership on Rare Diseases", was showcased by Daria Julkowska, which brings the European Commission and private and/or public partners together to address some of Europe's most pressing challenges through joint research and innovation initiatives. The program is planned to launch in 2024. The main goal of the collaboration is to improve the life quality of rare disease patients by developing new diagnostics and treatments.

Prioritizing rare disease funding

Rare disease research is unique in that there are limited numbers of patients spread all over Europe. The panelists called for prioritizing rare disease funding to expedite research and treatment options. Pernille Weiss, a policymaker responsible for the EU Pharma Package, further explained how budget allocations can significantly affect the progress of research and the affordability of treatments.

Apart from funding, the panel suggested incentives to make research more cost effective, e.g. lowering administrative burdens in hospitals or making more study nurses available.

These incentives, panelists agreed, need commitment from policy makers. As outlined by Alexander Natz, EUCOPE: "Rare diseases pose unique challenges, but they also provide incredible opportunities for innovation. We bridge the gap between academia, healthcare, and industry, and ultimately drive forward research that can transform lives."

The session was lead by moderator Peter Loeffelhardt, Global MPN Scientific Foundation, and expert panelists Susanne Greber-Platzer, Medical University of Vienna, Pernille Weiss, European Parliament, Daria Julkowska, European Joint Programme on Rare Diseases, and Alexander Natz, EUCOPE.

About AOP Health

The AOP Health Group incorporates several companies including AOP Orphan Pharmaceuticals GmbH with its seat in Vienna, Austria ("AOP Health"). The AOP Health Group is the European pioneer for integrated therapies for rare diseases and in critical care. Over the past 25 years, the Group has become an established provider of integrated therapy solutions operating from its headquarters in Vienna, its subsidiaries and representative offices throughout Europe and the Middle East, as well as through partners worldwide. The claim "Needs. Science. Trust." sums up the foundation of the Group's success: establishing trust through a continually high level of investment in research and development and a highly consistent and pragmatic orientation towards the needs of all stakeholders – especially the patients and their families as well as the healthcare professionals treating them.



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