

‘Rare Disease Moonshot’ – Scaling-up public-private partnerships to accelerate research into world’s rarest diseases



AMSTERDAM, Netherlands, December 8, 2022 — A new initiative to boost research and development into rare and paediatric diseases will launch today [Thursday 8 December] at the European Health Summit.

The ‘Rare Disease Moonshot’ is a commitment and collaboration between seven organisations to break down the barriers to finding new treatments and cures for the world’s rarest and severe conditions which currently have no therapeutic options, and which often affect the youngest patients.

The coalition will work together to pool expertise, reduce fragmentation in research and foster greater collaboration between organisations. It will bring together an ecosystem of rare disease experts and research to explore opportunities for collaboration and support a range of public-private partnerships to:



- Enhance translational research ecosystem to fill the research pipelines with new therapeutic options
- Optimise clinical trials and regulatory pathways for very small patient populations to de-risk and optimise development
- Develop infrastructure to accelerate the journey to diagnosis and treatment

The seven organisations are: the Critical Path Institute (C-Path); the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), the European Clinical Research Infrastructure Network (ECRIN), EuropaBio, EURORDIS-Rare Diseases Europe, the European Federation of Pharmaceutical Industries and Associations (EFPIA) and the European Infrastructure for Translational Medicine (EATRIS).

For decades, innovation has significantly advanced, with the emergence of new technologies like cell-editing and gene therapies. Between 2000 and 2021, more than 200 new orphan medicines were approved by the EMA, addressing the needs of up to 6.3 million rare disease patients. But significant unmet needs remain.

The coalition says it is vital to speed up research into more than 7000 identified rare diseases, of which a staggering 95 per cent have no approved treatment or cure. Experts say that at the current pace of R&D it could take more than 100 years to find treatments for all current rare conditions.

The Rare Disease Moonshot is also a response to the European Commission’s call to address unmet medical needs — and the belief that the science and translational capability gap in rare diseases cannot be addressed by one party or even one sector alone, nor can it be optimally served by a “patchwork” of unrelated initiatives. In order to improve the rare disease ecosystem in Europe, policy solutions need to be devised all along the lifecycle of medicines, from screening to patient access. This complex challenge calls for a vision

which captures the sum of public and private knowledge and capabilities to accelerate innovation – not only in joint undertakings, but also through vital funding initiatives at European and National levels, and in coordination with international initiatives. The Moonshot aims to boost the basic science and accelerate the translational research to address areas where no research is currently taking place.

If Moonshot is to succeed, Europe needs a solid pro-innovation ecosystem, supported by a stable intellectual property framework and a world-class regulatory system. While the journey to its shared goal of delivering new therapies for rare diseases where no treatment option exists is long, the coalition is confident this initiative can take us one step closer.



About Critical Path Institute

Critical Path Institute (C-Path) is an independent, global nonprofit established in 2005 as a public-private partnership. As a neutral convener, C-Path's mission is to be a catalyst for innovation that accelerates the path to a healthier world. Through collaboration, we develop new approaches to advance the development of new therapies and regulatory science. C-Path provides an open, precompetitive forum in which industry leaders, regulators, scientists, academic researchers and patient groups work together on solutions that stand to benefit those with the greatest need. C-Path offices are located in Tucson, Arizona and Amsterdam, Netherlands with additional staff in multiple other locations. For more information, visit c-path.org.

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