Press Release

Brussels – 11 June

European Expert Group on Orphan Drug Incentives unveils policy proposals to address unmet needs in rare diseases along the orphan drug development path

The multidisciplinary European Expert Group on Orphan Drug Incentives has developed fourteen innovative proposals along the entire lifecycle of orphan drug development to address the huge unmet needs in rare diseases. With over ninety percent of patients without a centrally approved treatment option, the need for innovation and research into orphan drugs is immense.

The OD Expert Group co-developed a report with policy proposals in a series of workshops and interviews with its knowledge partner Copenhagen Economics. They investigated how the current policy framework for Orphan Medicinal Products (OMP) incentives could be adapted to fit the unique challenges of the OMP development landscape, to the benefit and needs of rare disease patients.

At the launch of report of the OD Expert Group, one of the key drivers of the project, EURORDIS' Chief Executive Officer Yann le Cam stated: *"In proposing legislation that will shape the way we do things for the next twenty years, Europe must confront what works and what doesn't for people living with a rare disease.* With the revision of the EU Regulations on Orphan Medicinal Products and the future EU Pharma Strategy, Europe has a unique opportunity to foster investment in rare disease research and innovation - where it can bring the highest added value - paving the way for a new generation of highly innovative treatments. *The policy proposals from the Expert Group are another step in the right direction to have in place an ambitious European framework for rare diseases."*

In its report "Orphan Medicine Incentives. How to address the unmet needs of rare disease patients by transforming the European OMP landscape", the OD Expert Group makes a set of policy proposals that aims to improve the OMP incentive framework. The proposals range from basic research to patient access, considering the clinical development challenges, regulatory approval procedures and access mechanisms.

Reflecting on the initiative, coordinator of MetaBERN and co-chair of the OD Expert Group, Prof Maurizio Scarpa, pointed out that: *"The European Expert Group on OD Incentives is an excellent platform to discuss about drug evolution, drug legislation and drug availability in the space of Orphan Drugs. Patients need, more and more, efficient drugs, accessible and safe. Therefore, we have to ensure a system that works also beyond the revision of the current EU Orphan Drug framework. For me, the OD Expert Group is the right forum for such conversation and cooperation."*

The OD Expert Group's work is to shape the policy discussions around the OMP Regulation as well as the Pharmaceutical Strategy and inform policymakers about multi-stakeholders' perspectives.

For further information, visit the OD Expert Group's website: www.OD-ExpertGroup.eu

Or contact the team: carraro@eucope.org; simone.boselli@eurordis.org; gorfinkel@concilius.com

About the OD Expert Group

Established in 2020, the European Expert Group on Orphan Drug Incentives (OD Expert Group) brings together representatives of the broad rare disease community, including researchers, academia, patient representatives, members of the investor community, rare disease companies and trade associations. The initiative is led by a steering group composed of EURORDIS, the Voice of Rare Disease Patients in Europe and the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) representing several companies focused on finding new therapies for rare disease. The group is co-chaired by former MEP Renate Sommer and Professor Maurizio Scarpa, Coordinator of MetaBERN. The following OD Expert Group's members are sponsoring and providing expertise to the initiative: Alexion, Biogen, Bristol Myers Squibb, Chiesi, PTC Therapeutics, Takeda and EUCOPE.

Copenhagen Economics serves as Knowledge Partner to this initiative. The secretariat is led by CONCILIUS AG.