Press Release

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Multidisciplinary work of experts launched to bring forward innovative proposals for the next decades to foster research into new orphan drugs and therapies for rare diseases

In light of the upcoming Pharmaceutical Strategy and ongoing Evaluation of the Orphan Medicinal Products (OMP) Regulation, the European Expert Group on Orphan Drug Incentives has been established to discuss and bring forward innovative proposals to stimulate innovation and foster research into new orphan drugs and therapies for rare diseases.

In the driving seat of the project there are **EURORDIS,** the non-governmental rare diseases patient-driven alliance, and **EUCOPE**, the European association for small to medium-sized companies in the field of pharmaceuticals and medical technologies, many of which focused on rare diseases. Six EUCOPE member companies are providing expertise and financial resources to support the successful execution of the initiative: Alexion, Biogen, Bristol Myers Squibb, Chiesi, PTC Therapeutics and Takeda. The group counts on the leadership and expertise of two co-chairs, steering its activities along with the two organisations: **former MEP Renate Sommer** and **Prof. Maurizio Scarpa**, coordinator of MetaBERN and Chairman of the European Reference Networks Board of Coordinators (ERN-BC).

Reflecting on the work of the group, EURORDIS' Chief Executive Officer Yann le Cam said: *''After two decades of the Orphan Medicinal Products Regulation, we have witnessed great progress as well as important challenges both on development and access to therapies. The new European Expert Group on Orphan Drug Incentives allows for reflection on rare disease patients' opportunities for treatments: how can we take stock of progress that has been made over the past two decades, and ensure we move forward at pace so that no one is left behind?''*

The **multidisciplinary and cross-functional expert group brings together representatives of the broad rare disease community**, including research, academia, patient representatives, members of the investor community, rare disease companies and trade associations. This group aims to become the source of ground-breaking ideas and potential solutions that will provide input to the ongoing OMP Regulation Evaluation. The experts are currently working together to develop proposals to be unveiled by early 2021.

Commenting on the initiative and its comprehensive approach, EUCOPE Secretary General, Alexander Natz, stated: ''*We are at a crossroad. EU institutions, Member States, HTA bodies, payers, pharmaceutical industry and the scientific and rare disease community all share a responsibility to build on the achievements of the OMP Regulation. EUCOPE stands ready to contribute to this effort in cooperation with all relevant stakeholders, the European Expert Group on Orphan Drug Incentives is a unique opportunity to deliver on this shared goal*.''

The Expert Group is focusing on **three areas** to develop concrete policy proposals for potential improvements of the European OMP Incentives environment. These areas are:

- **Prioritisation and optimisation**: addressing therapeutic areas with currently no treatment and to support delivery of continued innovation for rare disease where treatments exist;
- **Regulatory pathways**: finding ways to optimize and accelerate regulatory pathways for orphan drugs;
- **Development incentives**: actions tailored to support OD development process, based on other international experience and ideas to trigger collaboration between academia, industry and regulators in Europe.

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

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