Joint Statement by EURORDIS and SIOPE on the Need for a First-in-Child Incentive in the EU

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EURORDIS – Rare Diseases Europe, a non-profit alliance representing over 1,000 rare disease patient organisations across Europe, and SIOPE – the European Society for Paediatric Oncology, representing over 2,700 healthcare professionals and researchers dedicated to childhood cancer, have joined forces to advocate for the specific needs of children with rare diseases and lack of targeted therapies.

We urgently call on policymakers to introduce a dedicated "first-in-child" incentive for drugs first developed specifically for paediatric use, to support orphan medicine development where it would otherwise be economically unviable.

Approximately 70% of rare diseases have their onset during childhood. Childhood cancers, all of which are classified as rare, remain the leading cause of disease-related death among children over the age of one in Europe—highlighting the urgent need for targeted incentives. However, most medicines are developed for adults, thus leading to significant challenges for treating children and adolescents: indications are often missing; age-appropriate formulations are not systematically available or adapted; and drugs are frequently administered off-label as they are developed for adult use. In childhood cancers for instance, such off-label use of anticancer drugs in children and adolescents can lead to both acute and long-term toxicities, causing a range – from mild to severe – of late side effects among survivors.

New orphan medicinal products for children —*particularly as a first authorised indication*—are needed to address the high unmet medical needs and health inequalities faced by a sheer number of young patients with a rare disease and their families. However, research in this area comes with exceptional challenges:

- clinical trials in children are logistically and ethically more complex;
- populations are extremely small and fragmented, making patient recruitment difficult;
- age-specific formulations and dosing considerations require additional research;
- there is a lack of commercial incentive due to limited market size and higher costs.

The current regulatory framework does not adequately support the development of orphan drugs for conditions that are unique to children. These diseases often have no adult equivalent, meaning the traditional model of adult-first development followed by a Paediatric Investigation Plan (PIP) does not apply. As a result, innovation for these high unmet needs in children remains economically unattractive and largely neglected.

SIOPE and EURORDIS strongly support a graduated incentive system that is adequate for such a highly underserved area as acknowledges the complexity of conducting research and development in the paediatric population.

Given the urgent need to develop targeted therapies for children and adolescents affected by a rare disease - including cancer - EURORDIS and SIOPE call for the introduction of a dedicated "first-in-child" incentive to promote and reward the complex development of orphan medicinal products intended for the paediatric population, where high unmet medical needs persist and where innovation is often economically unviable without tailored policy support. An additional one-year market exclusivity extension should be granted when an orphan drug is initially developed for a specific indication in children and adolescents with a rare disease.

With the General Pharmaceutical Legislation revision reaching its final steps, the window of opportunity for the EU to address this gap via a clear market exclusivity incentive for first-in-child Orphan Medicinal Products is closing. We call on co-legislators to reward first-in-child development for high unmet medical needs. In this way the EU would send a clear message that children—particularly those with rare and life-threatening diseases—deserve timely, safe, and effective treatments with the same urgency as adults.

About EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 1,000 rare disease organisations from 74 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe. By connecting people, families, and rare disease groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies, and services. Find out more: http://www.eurordis.org/

About the European Society for Paediatric Oncology (SIOPE)

The **European Society for Paediatric Oncology (SIOPE, or SIOP Europe),** is the single united European organisation representing all professionals working in the field of childhood cancers. With more than 2,700 members across 36 countries, SIOP Europe is leading the way to ensure the best possible care and outcomes for all children and adolescents with cancer in Europe. Find out more: https://www.siope.eu