Roadmap Response to Inception Impact Assessment on Medicines for Children and Rare Diseases Regulations

SIOPE and CCI-E welcome the proposed revision of EU regulations on medicines for children and rare diseases.

All paediatric cancers individually are rare, but jointly are the leading cause of death by disease in children above one year of age in Europe. Most survivors experience long-term side-effects due to older medicines. There are over 60 individual paediatric cancer types, each further sub-divided by stage, biology, and other factors.

As concluded by the evaluation of the Paediatric and Orphan regulations (OPR) "neither regulation has proven effective in boosting the development of innovative medicines for children with rare diseases, such as paediatric malignancies". A concurrent revision of the OPR has the potential to provide solutions to address unmet medical needs (UMN) of children with cancer and greatly enhance innovation.

We support the overarching objectives, in particular:

- Fostering research and development of medicines devised for adult cancer in the paediatric population and prioritizing products that address UMN
- Revising conditional exemptions from the obligation to study new medicines in children e.g. those with a relevant mechanism of action (MOA)
- An improved rewards system that would better help patients with UMN and ensure equal access to medicines for children with cancer across Europe
- Dedicated funding to academia and SMEs.

To accelerate innovation for children with cancer, we are strongly advocating for additional measures to achieve the following goals:

- Reduce delays in starting MOA-driven paediatric development of medicinal products
- Facilitate repositioning of unlicensed products failing in adults for the treatment of paediatric diseases, when there is a scientific and preclinical rationale.
- Incentivise 'First-in-Child' development and marketing authorisation for medicines against specific paediatric biological targets
- Introduce tailored and flexible incentives awarded incrementally and not only at the end of Supplementary Protection Certificate (SPC)
- Align regulations with other international jurisdictions, because drug development for rare paediatric diseases is global.

Identifying UMN will be paramount in the implementation of the revised regulations. Defining UMN in paediatric malignancies is complex as in other therapeutic areas. A fixed set of criteria established in the legislation would be detrimental and counterproductive. The definition of UMN should be dynamic and established in a multi-stakeholder setting. The legislation should provide a structure and framework where the needs can be continually identified and evaluated and the products prioritised.

MEDICINES FOR CHILDREN: SIOPE & CCI-E favour Option 3 (building on Option 1). We propose to incentivise starting paediatric development early by introducing changes to the timing and nature of rewards. A segmented approach with part delivered on completion of an interim deliverable would be a significant advance, while the remainder would be given on completion of the full PIP, as currently defined. For products addressing UMN, we support a novel reward that would complement or replace

the SPC prolongation. To this extent transferable vouchers are particularly attractive to incentivise First-in-Child development and development of medicinal products specific to paediatric diseases.

MEDICINES FOR RARE DISEASES: SIOPE & CCI-E favour Options 2 and 3. We fully support the use of incidence to qualify rare, including paediatric, cancers. There are many epidemiological registries that can provide relevant information on cancer incidence. Novel incentives would probably boost the development of products addressing UMN of rare paediatric diseases.

CONCLUSION: The European childhood cancer community is eager to contribute further and proposes the attached 6 Recommendations for Paediatric Cancers on access to medicines and innovation. These are also relevant to other paediatric life-threatening rare diseases.