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European Paediatric Cancer Community Proposal for Improvement of the European Biotech Act I

INTRODUCTION AND BACKGROUND

The **European Society for Paediatric Oncology (SIOPE),** the single-united European organisation representing all healthcare professionals in paediatric oncology, and **Childhood Cancer International – Europe (CCI-E)**, the largest European patient' organisation in paediatric cancer, welcome the European Biotech Act's ambition to accelerate innovation, improve competitiveness, and reduce time-to-market for advanced therapies. We believe it represents a timely and important initiative to strengthen Europe's capacity to deliver innovative, life-saving treatments for rare paediatric diseases.

The development of new medicines for these conditions has stalled significantly over the last decades due to complexities associated with small and specific high-risk patient populations. Over the last ten years, only 16 anticancer medicines have been authorised for a specific paediatric cancer indication, in contrast to over 150 for adult cancers[1]. Unfortunately, the review of the Pharmaceutical Package does not include specific indications targeting First-in-Child (FiC) drug development to significantly improve young cancer patients' chance of survival. Hence, we are calling for an ambitious Biotech Act I that will support and accelerate innovation and put the unmet medical needs of patients and their families at the centre of a European strategy for medicine development.

Patient recruitment remains a challenge in clinical trials design due to the heterogeneous and small size of the patient population eligible for clinical trials. This challenge contributes to an economically unattractive investment environment in this field. Therefore, we strongly recommend including a **FiC patent extension incentive**, as this would be expected to increase commercial interest in the development of medicines specific to rare paediatric diseases many of which are life-threatening.

We have **detailed below areas which should be upheld, as well as areas with specific suggestions for amendments** which can further strengthen the Act and ultimately, improve the outcomes of patients with unmet medical needs such as young cancer patients and restore them to their full health.

SUGGESTED AMENDMENTS AND SUPPORTED ARTICLES

➢ We advocate for the introduction of an additional **12-month period of patent extension** in Article 27 of the Biotech Act I Regulation for medicinal products **whose first authorised indication targets rare life-threatening paediatric diseases**.

➢ Besides, we call for EU programmes to **allocate public funds to research projects addressing such diseases**.

[1] Vassal et al., "Impact of the EU Paediatric Medicine Regulation on New Anticancer Medicines for the Treatment of Children and Adolescents, The Lancet Child & Adolescent Health, 2023 (https://www.thelancet.com/pdfs/journals/lanchi/PIIS2352-4642(22)00344-3.pdf).

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Art. 27: Extension of the supplementary protection certificate concerning best-in-class biotechnology medicines developed in the Union

Where a marketing authorisation is granted by the Union to a medicinal product for human use developed by means of biotechnological processes referred to in paragraph 1 of Annex I to Regulation (EU) …/… [reference to be added after adoption cf. COM(2023) 193 final] or to an advanced therapy medicinal product referred to in paragraph 2 of that Annex, and that is protected either by a supplementary protection certificate in accordance with Regulation (EC) No 469/2009 of the European Parliament and of the Council69, or by a patent which qualifies for the granting of such supplementary protection certificate, the holder of a patent or of such certificate shall be entitled to a 12-month extension of the periods referred to in Article 13, paragraphs (1) and (2), of Regulation (EC) No 469/2009, provided that the marketing authorisation applicant demonstrates that all of the following conditions are met:

(a) the medicinal product contains a new active substance distinctly different from that of any authorised medicinal product in the Union;

(b) the medicinal product has a mechanism of action distinctly different and shows a level of safety and efficacy which is at least equivalent to that of any authorised medicinal product in the Union for the same disease ***if any such medicinal product exists****;*

(c) the clinical trials evaluating the efficacy of the medicinal product and supporting its marketing authorisation were conducted in more than two Member States;

(d) at least a manufacturing step, excluding packaging, quality testing and certification is performed in the Union.

(New) Art. 27a:

Where the medicinal product addresses a rare life-threatening disease in children, the holder of the patent or certificate shall be entitled to an additional 12-month extension of the periods referred to in Article 13, paragraphs (1) and (2), of Regulation (EC) No 469/2009

In light of the above, we also propose to include '**non-commercial entities, including non-profit investigators**' in articles referring to providing 'support to promotors of biotechnology projects, SMEs, start-ups and scale ups', and support the current text in articles on data protection and Clinical Trials Regulations (CTR) on vulnerable populations to facilitate research:

Art. 1: Definitions

"This Regulation establishes a framework to strengthen the competitiveness of the health biotechnology sector in the Union. This Regulation lays down measures regarding:

(c) the support to promoters of biotechnology projects, SMEs, start-ups and scale ups, ***and non-commercial entities, including non-profit developers,*** *of biotechnology products, by establishing an EU Health Biotechnology Support Network;*

(48) 'Art. 93: Data protection

6. "Personal data collected and processed in accordance with this Regulation may be further processed by the same controller for the purposes of other clinical trials conducted under this Regulation, or for scientific research with the aim of protecting public health, improving standard of care and fostering the innovation capacity of European medical research.

7. By derogation from Article 9(4) of Regulation (EU) 2016/679, Member States may not maintain or introduce further conditions, including limitations, with regard to the processing of personal data, including genetic data or data concerning health in the context of clinical trials carried out in accordance with this Regulation.

Art. 58: Amendments to Regulation (EU) No 536/2014

(9) "In Article 10, the following paragraph 6 is added:

'6. Where potential subjects of a clinical trial belong to vulnerable populations, Member States concerned and sponsors shall consider and weigh the harms and benefits of their inclusion as opposed to their exclusion from a clinical trial. The Member States concerned and sponsors shall assess in particular whether the exclusion of those subjects from a clinical trial could inadvertently perpetuate or exacerbate their vulnerabilities, particularly in relation to their specific health needs.

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ABOUT EUROPEAN CHILDHOOD CANCER ORGANISATIONS

Childhood Cancer International - Europe (CCI-E, or CCI Europe) represents childhood cancer parent and survivor groups as well as other childhood cancer organisations in Europe: 67 organisations in 34 European countries are members of CCI-E. CCI Europe works together with all relevant stakeholders for the same aim: help children and adolescents with cancer to be cured, with no - or as few as possible - long term health problems/late effects. **(www.ccieurope.eu)**

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 2,500 members across 35 countries, SIOP Europe is leading the way to ensure the best possible care and outcomes for all children and adolescents with cancer in Europe. **(www.siope.eu)**