ACCELERATE CONTRIBUTION TO THE EU INCEPTION IMPACT ASSESSMENT ON PROPOSED REVISION OF PAEDIATRIC AND ORPHAN REGULATION

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ACCELERATE, the international multi-stakeholder platform which aims to accelerate innovation in drug development for children and adolescent with cancer, welcomes the initiative of the European Commission to revise both the Paediatric and Orphan Regulations. Indeed, the unmet medical needs of children with cancer have not been appropriately addressed to date, while science-driven innovation and research and development (including precision oncology, immunotherapy, and targeted therapies) have profoundly changed and impacted the therapeutic landscape of adult cancers.

ACCELERATE has demonstrated the value of multi-stakeholder cooperation, with representatives from academia, industry, regulatory agencies (EMA, FDA and agencies from other jurisdictions) and patient advocates collaborating to improve and accelerate the development of innovative therapies for children with cancer. Improving access to innovative medicines is also essential and should be addressed early in paediatric drug development plans; ACCELERATE aims to work with Health Technology Assessment agencies to achieve this goal.

ACCELERATE supports the proposal to foster science-driven research and development of medicines devised for adult cancer in the paediatric population (including revising conditional exemptions to consider drug mechanism of action rather than adult condition) and prioritising products that address unmet medical needs. Unmet medical needs should be defined in a multi-stakeholder setting rather than through fixed criteria in the regulations.

ACCELERATE has demonstrated the feasibility and value of a multi-stakeholder initiative through the development of Paediatric Strategy Forums with EMA in collaboration with FDA to define unmet therapeutic needs in paediatric malignancies and to facilitate prioritisation. The legislation should provide a structure and framework where multi-stakeholder cooperation would be paramount to continually identify and evaluate unmet needs and prioritise medicinal products.

Improving the system of incentives and rewards is required. The revision should build on the successes achieved so far rather than completely replacing the current system. Better tailored and optimised incentives should be established to reward early start of paediatric development and evaluation of products addressing unmet needs. An ACCELERATE multi-stakeholder working group has proposed to define interim and final deliverables within the Paediatric Investigation Plan (PIP), each attracting a reward on completion.

There are numerous experimental medicinal products that are “shelved” after their development in adults is terminated, not always for lack of efficacy or safety concerns. Facilitating science-driven repurposing of shelved experimental drugs for paediatric development would contribute to accelerate innovation for children with cancer and other life-threatening diseases. In addition, novel rewards, such as transferable vouchers, would incentivise both the development of paediatric
medicines addressing the specific biological alterations of paediatric disease (first in child product development) and approval of products for unmet needs.

ACCELERATE highlights that international cooperation is paramount because each paediatric cancer is individually rare and developing innovative therapies is necessarily a global endeavour.

ACCELERATE is currently working to identify current barriers for academic and industry sponsored international trials. FDA, EMA and agencies from other jurisdictions have already started to work together in the paediatric cluster calls. Reinforcing cooperation towards greater alignment is needed to accelerate approval of the global science-driven paediatric development of medicinal products that will address unmet therapeutic needs of children suffering from cancer or other rare life-threatening diseases.