ACCELERATE, the international multi-stakeholder platform which aims to accelerate innovation in drug development for children and adolescent with cancer, welcomes the revision of the general pharmaceutical legislative framework in the scope of the pharmaceutical Strategy for Europe. ACCELERATE agrees with the European Commission that there is a need to address unmet medical needs and market failures for non-orphan and paediatric medicines. The unmet medical needs in childhood cancer have not been properly addressed to this day, while the innovation, research, and development in the therapeutic area of adult cancers has evolved drastically (introduction of precision oncology, immunotherapy, and targeted therapies).

ACCELERATE shows the value of multi-stakeholder cooperation, bringing together representatives from academia, industry, regulatory agencies (EMA, FDA and agencies from other geographical jurisdictions worldwide) and patient advocates working together to improve and accelerate innovative therapies for childhood cancer patients. In line with the objective of ensuring access to affordable medicines in the scope of this impact assessment, ACCELERATE acknowledges that it is also important to improve access to innovative medicines.

We believe unmet medical needs should be defined in a multi-stakeholder setting rather than through criteria-based regulation. 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. The pharmaceutical legislation should enforce a structural framework where unmet medical needs would be continuously identified and evaluated through multi-stakeholder collaborations and the latter would further prioritize medicinal products from these evaluations. We believe prioritisation of oncology drugs should be established in a multi-stakeholder setting to match unmet medical needs in children with cancer to the best available therapies.

ACCELERATE endorses the revision of the system of incentives to attract and promote innovation, especially in areas of highest medical need such as childhood cancers. The revision should build on past achievements rather than completely replacing the current system. We believe better tailored and optimised incentives should be established to reward early start of paediatric medicines development, addressing unmet needs. Within an ACCELERATE multi-stakeholder working group, the representatives proposed to define interim and final deliverables to attract a reward on completion of the paediatric investigation plan.

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.

Ways to increase and accelerate product development and authorisation in areas of unmet need, ACCELERATE highlights that there is a variety of experimental medicines that are ‘shelved’ after their
development is terminated, but not because of lack of efficacy or safety issues. Facilitating science-driven repurposing of ‘shelved’ experimental medicines would contribute to accelerate innovation to address unmet medical needs.

Finally, ACCELERATE highlights that multi-stakeholder, international collaboration 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.

Reinforcing cooperation towards greater alignment is needed to accelerate approval of the global science-driven paediatric development of medicinal products to address unmet therapeutic needs of children suffering from cancer or other rare life-threatening diseases.

Link: https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12963-Revision-of-the-EU-general-pharmaceuticals-legislation_en