

New U.S. Law to Improve International Research for Childhood Cancer

Children with cancer will have substantially increased access to promising new therapies as a result of the **RACE for Children Act**, a U.S. law launched in August 2020. Years in the making, this legislation will drastically alter the international drug development landscape for children with cancer.

Prior to enactment of the law, children did not have access to new drugs until after their approval for adult cancer treatment. U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) regulations prevented children from having early access to new therapies because drug approval was based on adult diseases, such as breast and lung cancer, which do not occur in children. Rather than using the physical site of a cancer, the RACE for Children Act established a different standard: companies are now *required* to determine whether an adult cancer drug's biological "mechanism of action" is molecularly relevant in a childhood cancer. Companies can no longer dismiss as irrelevant new adult cancer agents with potential for treating childhood cancer.

Implementation of the RACE Act is resulting in increased support for preclinical and clinical research, both of which are advancing international efforts to improve childhood cancer treatment. Preclinical research is critical to determining whether new chemical compounds target critical biological vulnerabilities in childhood cancers and whether these compounds can be turned into medicines for children. Two wide-ranging programs, the EU's [ITCCP-4](#) (Paediatric Preclinical Proof of Concept Platform) and a planned U.S. public-private-partnership through the Foundation for the National Institutes of Health, will yield essential preclinical data on childhood cancers. Information from cell lines and animal models of childhood cancer, for example, will be necessary as companies evaluate their new agents to meet FDA requirements and EMA review.

Recent research has revealed many subtypes of childhood cancers, resulting in decreased numbers of eligible children in which to evaluate relevant targeted therapies. More drugs and fewer patients also means that priorities must be set among multiple drugs with the same mechanisms of action. Researchers, regulators and advocates are addressing this critical challenge through [ACCELERATE](#), an international platform for innovation in drug development for children and adolescents with cancer. Stakeholders discuss and recommend which new agents should be tested for which diseases in transparent, deliberative, scientific "Pediatric Strategy Forums".

With more drugs available and fewer eligible patients for each trial, international clinical trials will be more important not just to have enough patients for valid trials, but also to bring new therapies to children globally.

ACCELERATE Team

contact@accelerate-platform.org

Relevant publication on the implementation of the RACE for Children Act:

- Pearson ADJ et al, *The RACE to accelerate drug development for children with cancer*, THE LANCET Child and Adolescent Health. 2020; Vol. 4, Issue 10, 714-716

With the support of

