

RACE Act increased the number of cancer drugs with required studies for use in pediatric patients

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In its first year of implementation, the RACE Act has resulted in increased requirements for more recently approved cancer drugs to be

studied for potential use in pediatric patients, according to data presented at the AACR Annual Meeting 2022, held April 8-13.

The Research to Accelerate Cures and Equity (RACE) for Children Act was approved by the U.S. Congress in 2017 and took effect on August 18, 2020. The act requires all new adult oncology therapeutics under consideration for approval by the U.S. Food and Drug Administration (FDA) to be evaluated for safety and efficacy in [pediatric cancers](#) if the treatment is directed at a molecular target relevant to pediatric cancer, including therapeutics with an orphan drug designation.

"Significant progress has been made in [cancer care](#) and treatment, with many novel therapeutics recently approved. Many of these exciting advancements, however, have not easily translated to new treatment options and approvals for pediatric oncology patients," said the study's lead author, Brittany Avin McKelvey, Ph.D., science policy analyst with Friends of Cancer Research.

Prior to the RACE Act, [pharmaceutical companies](#) were not required to conduct [clinical trials](#) of their product in pediatric populations if the product was intended for use in an adult cancer. Also, any drugs that received an orphan drug designation were automatically exempt from conducting pediatric studies, even if they had potential applicability to pediatric cancers, McKelvey said.

In this study, the researchers sought to evaluate the impact of the RACE Act in its first year of implementation. They examined all new oncology drug applications or biologics license applications approved by the FDA from August 18, 2019, to August 18, 2021. Nineteen therapeutics were identified within the study period; 63.2 percent were approved pre-RACE implementation and 36.8 percent were approved post-RACE implementation. McKelvey noted that the lower number of approvals in the second year may have been due to the coronavirus pandemic; further

study will be necessary to assess the continuing effects of the RACE Act.

In the pre-RACE group, 8.3 percent of the [approved drugs](#) were indicated for pediatric use at the time of application. However, all of the drugs approved in the pre-RACE group were deemed exempt from pediatric studies or had the requirement waived. Alternatively, in the post-RACE group, 42.9 percent of the approved drugs have been required to conduct pediatric studies.

Prior to the RACE Act, 83.3 percent of the approved drugs in the study received an orphan drug designation, meaning that the drugs were exempt from required pediatric studies. McKelvey said that nearly all of them (91.7 percent) had a mechanism of action that may have required pediatric studies if the application had been submitted after the RACE Act was in effect.

After the RACE Act, 71.4 percent of the approved drugs received an orphan drug designation, but 60 percent of those were required to conduct pediatric studies because their mechanism of action was considered relevant to pediatric cancer, and therefore, the drug could be potentially beneficial to pediatric patients.

McKelvey shared one concrete example of how the RACE Act is resulting in more studies for pediatric patients. In April 2020, shortly before the RACE Act was implemented, the FDA approved pemigatinib (Pemazyre), a kinase inhibitor used to treat patients with cholangiocarcinoma, a rare bile duct cancer. The drug, which targets an FGFR2 alteration, received an orphan designation, meaning that pediatric study was not required.

Just over a year later, in May 2021, the FDA approved infigratinib (Truseltiq), which also targets FGFR2 alterations. The drug also received

orphan designation, but because of the RACE Act, it must now be studied in pediatric patients with advanced or metastatic solid tumors with FGFR2 alterations.

"We were excited to see, even in the first year after implementation, a significant increase in required pediatric studies—especially the significant effect of closing the orphan designation loophole," McKelvey said.

Because many childhood cancers are rare, researchers expect that clinical trials may progress slowly as researchers work to accrue eligible patients. McKelvey cited two trials that have launched since the RACE Act that have set completion dates of 2028, noting that these may point to the need for innovative trial designs and additional mechanisms to support pediatric study.

"We still have many years until we will be able to see if the required pediatric studies lead to actionable data that can support labeling modifications and inclusion of information for appropriate use in pediatric patients," McKelvey said. "The first step, which RACE has helped achieve, is to encourage study of novel adult therapies in pediatric patients. The long-term measure of success will be if these studies result in patient benefit and approval for pediatric patients."

"As a pediatric [cancer](#) survivor myself, I recognize that the RACE Act was an important step forward in increasing pediatric studies of novel therapeutics and encouraging innovation," McKelvey continued. "But implementation is only the beginning. It is now crucial to follow the impact of the act on pediatric oncology drug development and continue to strive for policy and regulatory solutions so that [pediatric patients](#) have access to lifesaving therapies and information to support treatment decisions."

McKelvey said the primary limitation of the study is the limited number of therapeutics included in the initial analysis one year after the RACE Act took effect. She said further longitudinal data will be necessary to fully assess the impact of the RACE Act and determine future policy recommendations.

More information: Conference:

www.aacr.org/meeting/aacr-annual-meeting-2022/

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