AAP News™

Drug development for pediatric cancers accelerating

March 1, 2023

from the Food and Drug Administration

Article type: FDA Update

Topics: Hematology/Oncology, Pharmacology, Therapeutics

Ensuring children with cancer have safe and effective treatment options is a key priority for the Food and Drug Administration (FDA).

Since the early 1950s, the FDA has approved more than 50 drugs with over 70 indications for the treatment of pediatric cancers. More than 30 of these drugs were approved in the last 10 years. The number of drugs approved has grown substantially in the last two decades because of laws that help accelerate drug development for children and as knowledge about tumor biology has increased.

“We’re seeing the landscape for pediatric oncology drug development expand dramatically,” said Martha Donoghue, M.D., associate director for pediatric oncology and rare cancers in the FDA’s Oncology Center of Excellence.

Newer therapies that target specific molecules involved in the growth or spread of cancer cells have been a key driver for the recent progress in cancer treatment. In contrast to older cytotoxic chemotherapy drugs,

molecularly targeted products may cause less harm to healthy cells.

Although traditional chemotherapy and radiation remain mainstays of cancer treatment, a variety of therapeutic agents now are part of the growing pediatric oncology armamentarium. For example, small-molecule kinase inhibitors, monoclonal antibodies and other immune-based therapies like genetically engineered cell therapy (CAR-T cells) have been approved for pediatric patients across a wide range of pediatric cancers.

“We are committed to helping ensure children with cancer are not left behind as we enter new frontiers in cancer treatment,” Dr. Donoghue said. “As one example, the first gene therapy approved by the FDA was for treatment of pediatric and young adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia.”

Federal laws have been important to help ensure medicines are developed for children with cancer. Most recently, the Research to Accelerate Cures and Equity (RACE) for Children Act of 2017 gave the FDA authority to require the pharmaceutical industry to submit reports on pediatric cancer investigations for certain new molecularly targeted products under development for the treatment of adult cancers. For example, if a new drug intended for treatment of breast cancer has a target that is relevant to the growth or progression of a pediatric cancer, the FDA can require a study evaluating dosing, pharmacokinetics, safety and preliminary efficacy in pediatric patients with cancer.

Early evidence suggests that the RACE Act has improved drug companies’ timely consideration and initiation of pediatric studies of appropriate molecularly targeted cancer drugs that historically would have gone unstudied in children (https://www.gao.gov/products/gao-23-105947).

“The FDA will continue to work collaboratively with all stakeholders in the pediatric oncology community to facilitate the timely and efficient development of drugs that meet the needs of children with cancer,” Dr. Donoghue said. “We’ve issued several guidance documents to address important considerations in pediatric oncology drug development, including how to develop pediatric study plans for molecularly targeted drugs, age considerations for clinical trial enrollment, inclusion of adolescent patients in adult clinical trials and use of master protocols.”

The FDA’s Office of Pediatric Therapeutics, Oncology Center of Excellence and Office of New Drug’s Division of Pediatrics and Maternal Health contributed to this article.

Resources

- Information on pediatric oncology at the FDA
- Drugs approved for pediatric cancers
- FDA guidance documents