Drug development for rare diseases affecting children is increasing

March 3 2020

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The number of treatments for rare diseases affecting children has increased, a new study suggests. But federal incentives intended to encourage drug development for rare conditions are being used more often to expand the use of existing drugs rather than for creating new ones.

Children are estimated to make up half of patients with rare diseases, which includes conditions that affect less than 200,000 Americans. Developing drugs for children with rare diseases is crucial to ensuring new treatment options, but it's not always profitable for drug manufacturers.

To motivate manufacturers to develop drugs to treat rare diseases, or "orphan drugs," federal policy provides incentives such as tax credits, grants for testing, and a seven-year period of exclusivity during which competitors can't market an alternative version of the drug for the same disease.

Of the 402 orphan drug indications approved through the U.S. Food and Drug Administration between 2010 and 2018, a third were specifically for children or for diseases that predominantly affected children, according to the findings published in *Pediatrics*.

But most of these pediatric orphan drug approvals were new uses of existing drugs, some of which are decades old and had already been approved to treat common diseases. Twenty received breakthrough designation, which is granted to drugs that hold particular promise for improving upon existing treatments.

"Our study reveals reason for optimism and reason for concern," says senior author Kao-Ping Chua, M.D., Ph.D., a pediatrician and researcher at Michigan Medicine C.S. Mott Children's Hospital and the Susan B. Meister Child Health Evaluation and Research Center.

"Many pediatric orphan indications may have represented breakthroughs for children with rare diseases. At the same time, most indications were not for new drugs, and some represented relatively minor expansions of use. Orphan drugs are costly to society, and it's important to make sure that these costs are justified by the amount of benefit to patients."

The researchers found that the 136 pediatric orphan drug approvals targeted 87 unique diseases, most commonly cystic fibrosis, acute lymphoblastic leukemia and immune disorder hereditary angioedema.

"Although the Orphan Drug Act has been effective in incentivizing drug development, our findings suggest that not all pediatric orphan indications hold the same value," says lead author Lauren Kimmel, a research assistant at the University of Michigan Medical School and CHEAR.

"Policymakers should ensure that resources are being used efficiently and effectively to stimulate development of new therapies for rare diseases that don't have any treatment options."

More information: Lauren Kimmel et al, Pediatric Orphan Drug Indications: 2010–2018, *Pediatrics* (2020). DOI: 10.1542/peds.2019-3128

Provided by University of Michigan