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FDA working to advance development of drugs for pediatric heart failure

from the Food and Drug Administration Office of Pediatric Therapeutics, Division of Cardiovascular and Renal Products, and Division of Pediatric and Maternal Health

The Food and Drug Administration (FDA) recognizes the need for safe and effective therapies for pediatric patients with heart failure.

While more than 20 drugs have been approved to treat heart failure in adults, none are approved for pediatric patients. Furthermore, only one drug for pediatric heart failure has been studied under the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act, and it failed to demonstrate efficacy.

Therefore, the FDA and the University of Maryland Center for Excellence in Regulatory Science and Innovation recently hosted a public workshop to identify potential paths to successful treatment trials in pediatric heart failure.

The workshop addressed challenges related to the evaluation of products, including choice of study population and selection of appropriate endpoints. Speakers also noted that the etiologies of pediatric heart failure in children differ from those in adults, making it difficult to extrapolate data from adults.

Stakeholders from regulatory agencies, academia and industry considered various trial designs that may allow for the use of short-term outcome measures. They discussed use of biomarkers, external controls, quantitative systems pharmacology and Bayesian decision-making to optimize pediatric trials. Data from registries (e.g., Pediatric Heart Network, Pediatric Cardiomyopathy Registry) also may facilitate understanding of the natural history of pediatric heart failure and help identify feasible clinical trial designs.

Representatives from the European Medicines Agency offered their views on the development of drugs for pediatric heart failure, and an adult patient provided the perspective of someone who was diagnosed with the disease as an infant.

The FDA plans to publish the workshop proceedings and will continue to work with stakeholders to advance drug development in pediatric heart failure.

Resources

- [Slides and video from the workshop](#)
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