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Forum encourages dialogue on rare disease programs

by from the Food and Drug Administration's Division of Pediatric and Maternal Health, Division of Gastroenterology and Inborn Errors, Center for Drug Evaluation and Research, and Office of Pediatric Therapeutics

The first meeting of the Rare Diseases Forum drew over 100 stakeholders, who discussed how to facilitate drug development for rare diseases.

The forum is a coordinated effort among the Food and Drug Administration (FDA), the Forum for Collaborative Research of the University of California Berkeley School of Public Health, industry, the academic community and patient advocacy groups.

The goals of the forum include enhancing communication and collaboration, maximizing efficiency, and encouraging the development of innovative analytics with novel biotechnology.

Highlights of the first forum in October 2018 included three case presentations on recently approved products: Palynziq for adults with phenylketonuria, Brineura for children with late infantile neuronal lipofuscinosis type 2 and Mepsevii for patients with mucopolysaccharidosis type VII.

The case presentations provided a platform for industry and the FDA to provide their assessments on the development of each drug, commenting on the strengths and challenges of the individual programs. This unique setting encouraged stakeholder dialogue from a variety of perspectives, including patient-focused development, the regulatory decision process and the impact of the 21st Century Cures Act on rare disease programs.

In the group discussions, the FDA encouraged early communication in drug development programs for the selection of disease relevant endpoints and appropriate study duration, in addition to the importance of obtaining consistent, interpretable data that may facilitate regulatory flexibility.

Planning for the 2019 forum is underway.

Resources

- Information on the Rare Diseases Forum
- Additional FDA Update columns