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FDA seeks pediatrician input on therapeutic development for children

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Food and Drug Administration

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Editor's note: *The FDA's public meeting was changed from in-person to virtual after the April issue of* AAP News *went to print.*

The Food and Drug Administration (FDA) is holding a virtual public meeting on May 15 for pediatricians, health care professionals who work with children, industry representatives and patient and caregiver groups to give feedback on implementation of the Best Pharmaceuticals for Children Act (BPCA) and Pediatric Research Equity Act (PREA).

BPCA and PREA were passed more than two decades ago to encourage and require studies of drugs and biological products for use in children.

"Hearing perspectives from pediatric providers is essential for us to understand the needs of the pediatric clinical and research communities so we can continue to drive therapeutics development for children," said Dionna Green, M.D., director of the FDA's Office of Pediatric Therapeutics.

Some issues to be discussed at the meeting include:

- the public health impact the laws may have had on interested parties or their communities, including treatment advances and areas of continued unmet medical need for pediatric patients;
- barriers or resource issues preventing undertaking or completing pediatric studies, including issues related to clinical trial infrastructure and enrollment, and ensuring pediatric clinical trial populations reflect the community of children most likely to benefit from the therapeutics;
- how the laws' requirements and incentives for pediatric studies affect the drug development enterprise; and
- successes and challenges with leveraging scientific advances in drug development, including use of pediatric extrapolation, adaptive trial designs, biomarkers as surrogates and real-world data, to facilitate more timely evidence generation for pediatric populations.

The FDA is preparing to submit its third report to Congress in 2026 to provide an assessment of the implementation and impact of these laws. The report also highlights successes and describes potential improvements for advancing pediatric drug development. Input from interested parties will be incorporated into the report.

The meeting will be held from 9 a.m. to 4:30 p.m. ET on May 15 as a virtual webinar for registered participants. Participants may submit a request to make oral comments at the meeting by emailing OPT@fda.hhs.gov by May 1.

Regardless of attendance at the public meeting, individuals can submit comments to the FDA via public docket number FDA-2024-N-5784 at https://www.regulations.gov. Comments will be accepted until 11:59 p.m. ET on June 13.

"We hope you'll join us at the meeting and provide your feedback, whether through oral comments or by submitting a statement to the docket," Dr. Green said.

The FDA's Office of Pediatric Therapeutics and Office of New Drug's Division of Pediatrics and Maternal Health contributed to this article.

Resources

- Register for the Interested Parties Meeting: Implementation of the Best Pharmaceuticals for Children Act and Pediatric Research Equity Act.
- Submit comments to the FDA via public docket number FDA-2024-N-5784.
- FDA's past reports to Congress on BPCA and PREA.