Biotechnology Industry Organization

April 24, 2015

Dockets Management Branch (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Re: Docket No. FDA–20115–N–0456: Pediatric Stakeholder Meeting; Request for Comments

Dear Sir/Madam:

The Biotechnology Industry Organization (BIO) thanks the Food and Drug Administration (FDA) for the opportunity to present our views on pediatric drug development at the March 25 pediatric stakeholder meeting and to submit written comments to the docket. BIO applauds the leadership of the FDA for convening the stakeholder meeting to help foster collaboration and coordination across all pediatrics stakeholders, including industry, academia, the patient advocacy community, and government, so that we can work together to develop safe and effective new therapies for children.

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO members are involved in the research and development of innovative healthcare, agricultural, industrial and environmental biotechnology products.

A. General Comments:

BIO believes that BPCA and PREA have been successful in promoting a regulatory environment where medications used in children are tested and labeled appropriately for their use. BIO supports the need for appropriate studies of new biologics in pediatric patients as a way to improve and increase the treatment options for pediatric patients and their families.

In the two and a half years since Congress reauthorized the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act, the permanency of the pediatrics programs has provided biopharmaceutical companies with the confidence and predictability necessary to invest in the scientific capacity and infrastructure to advance pediatric research. Rather than balancing BPCA incentives on one hand and PREA requirements on the other, industry is adopting comprehensive and integrated approaches to pediatrics drug development in a manner that would not be possible without a permanent program.

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Yet there remains work to be done to continue to advance pediatric clinical research. We look forward to collaborating with FDA and other stakeholders to build on that record of accomplishment.

BIO thanks FDA for the many useful draft guidances produced since pediatric legislation was first enacted, and we urge the Agency to finalize these draft guidances as soon as possible. We also look forward to providing thoughtful feedback and comments to the Agency on future guidance development.

BIO appreciates FDA's sharing of data regarding compliance with post-approval pediatric commitments. The high level of compliance reflects the seriousness with which industry takes these commitments.

BIO looks forward to continuing a dialogue on certain aspects of the BPCA program, including variability in the review time of Proposed Pediatric Study Requests (PPSRs) and proposed amendments to written requests.

B. BIO Survey on the PSP Process

The pediatric study plan (PSP) process appears to be working well in the two years the process has been in effect. To better understand industry's experience with the process, BIO has undertaken a survey of its members to evaluate what in the PSP process is working well and where there may be room for improvement.

BIO anticipates having this data by June 2015 and plans to share it with FDA and other stakeholders in July 2015.

Regarding the PSP amendment process, BIO would be interested in hearing more about FDA's plans, as this process was not fully developed in the PSP draft guidance.

C. Permanency of the Pediatric Priority Review Voucher Program

BIO appreciates FDA's implementation of the rare pediatric disease priority review vouchers (PRVs) in a manner that stimulates new drug development of new therapies for devastating childhood diseases and serious conditions through additional incentive mechanisms. Now that the third voucher has been issued and the twelve month clock has begun to tick, BIO calls on Congress to make this successful program permanent.

BIO notes with pride that two of the three the pediatric priority review vouchers issued to date have been for biologics.

Given the industry interest in pediatric PRVs and their potential value as an incentive to spur pediatric research, we believe that the arbitrary cap on rare disease PRVs should be lifted. The limitation of three vouchers introduces significant uncertainty and unpredictability for sponsors who are considering the risky, long, and costly investment into a clinical development program for rare pediatric condition, especially if the twelve month period subsequent to the issuance of the third voucher has expired by the time of

FDA filing. BIO looks forward to working with Congress to make the program permanent, similar to the tropical disease PRV program.

D. Conclusion

Thank you for the opportunity to present our views on how we can further advance pediatric research and encourage the development and appropriate labeling of therapies for children. We would be pleased to provide any additional information, as needed.

Sincerely,

/S/

Andrew J. Emmett
Managing Director, Science and Regulatory Affairs
Biotechnology Industry Organization (BIO)