

Pediatric Drug and Device Laws: Reauthorization Summary

Food and Drug Administration Safety and Innovation Act
Enacted July 9, 2012

American Academy of Pediatrics
DEDICATED TO THE HEALTH OF ALL CHILDREN™



PEDIATRIC DRUG PROVISIONS

Permanently Reauthorizes Pediatric Drug Laws

- The *Best Pharmaceuticals for Children Act* (BPCA) and the *Pediatric Research Equity Act* (PREA) are reauthorized without sunset, becoming a permanent part of the Food, Drug, and Cosmetic Act and giving children a permanent seat at the drug development table.

Requires Earlier and Better Pediatric Study Planning

- Moves pediatrics earlier in the drug development process by requiring that drug companies submit pediatric study plans at the end of Phase 2. Plan content and timing closely modeled after the Pediatric Rule.
- Requires the FDA and drug companies to meet to discuss pediatric studies.
- Requires the FDA to publish, within one year, a proposed rule detailing the pediatric study planning process.

Adds New Enforcement Tools to Ensure Timely Pediatric Data Submission

- Requires the FDA to issue public non-compliance letters to companies that do not fulfill their PREA requirements on time.
- Allows companies to request an extension for good cause if PREA studies are delayed.

Increases Focus on Neonatal Drug Studies

- Requires all written requests issued under BPCA to include studies in neonates or to outline the rationale for not doing so.
- Requires that a neonatologist sit on the FDA's internal Pediatric Review Committee (PeRC).
- Requires the FDA to hire a neonatologist for at least five years to assist the agency in the appropriate application of BPCA and PREA to the neonatal population.
- Requires the FDA to hire an epidemiologist to assist with pediatric safety reviews.

Increases Transparency

- Requires the FDA to release on its website certain data reviews of BPCA studies submitted between 2002 and 2007 that have never been made publicly available.
- Improves content and timeliness of PREA annual reviews that must be made available on FDA's website.

Improves Accountability

- Clarifies that pediatric exclusivity cannot be granted for required PREA studies unless included in an FDA-issued written request.
- Requires that the PeRC review all initial and agreed pediatric study plans as well as any significant amendments to the plans or written requests. Also requires PeRC review of deferral extension requests.
- Requires the FDA to publish a publicly available report on BPCA and PREA every five years.

Other Improvements

- Renews the authorization of appropriations for the NIH BPCA program for five years at \$25 million per year.
- Eliminates unnecessary steps before the FDA may consider whether to require studies under PREA that were not completed under BPCA.
- Ensures that FDA can include any important pediatric safety information on certain generic drug labels.
- Clarifies that biologics approved under the Public Health Service Act can be included in the NIH BPCA program.
- Requires the FDA to hold a public meeting on accelerating development of therapies for pediatric rare diseases and develop a strategic plan.
- Permanently extends the operation of the Pediatric Advisory Committee and the Pediatric Subcommittee of the Oncologic Drugs Advisory Committee.

PEDIATRIC DEVICE PROVISIONS

Reauthorizes and Preserves Pediatric HDE Profit Incentive

- Expands profit allowance to adult-only devices approved under the humanitarian device exemption (HDE), while preserving the pediatric HDE profit *allowance*. Allows adult-only devices to qualify for the profit incentive if they are intended to treat or diagnose a disease or condition that either does not occur in children or “occurs in pediatric patients in such numbers that the development of the device for such patients is impossible, highly impracticable, or unsafe”.

Requires Final Rule on Pediatric Device Tracking

- To implement the pediatric device tracking provision from the 2007 law, requires the FDA to publish a proposed rule by December 31, 2012 and a final rule by December 31, 2013. This will require companies to submit pediatric information in device applications.

Reauthorizes the Pediatric Device Consortia

- Reauthorizes the successful Pediatric Device Consortia for five years at \$5.25 million per year.