Pediatric Stakeholder Meeting: **Continuing Progress on Drugs for Children**

Bridgette Jones, MD, FAAP
Chair, AAP Committee on Drugs

November 21, 2019

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

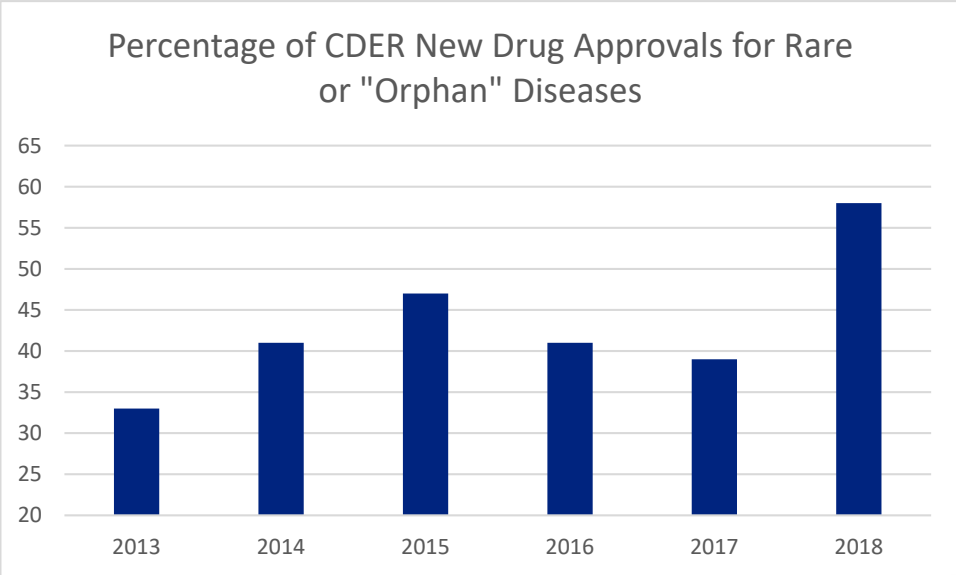
BPCA and PREA

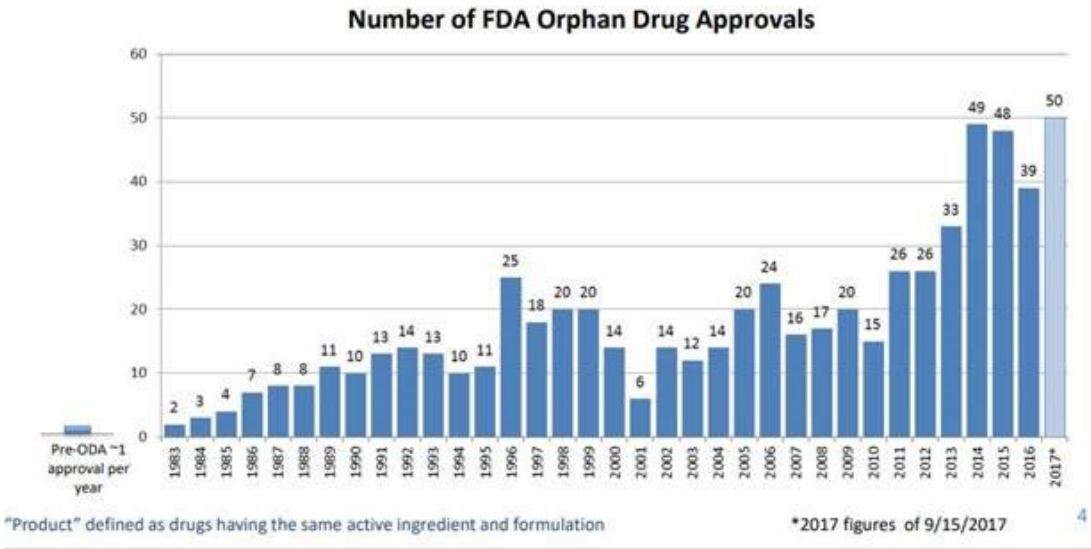
- The Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA) have revolutionized pediatric therapeutics.
- More than 800 pediatric label changes have been made as a result of BPCA and PREA.

- BPCA and PREA made permanent in 2012 giving children a permanent seat at the drug development table.

Orphan Drugs and PREA

- Orphan drugs are exempt from PREA requirements.
- Orphan drug approvals are increasing, and therefore, so are the number of new drugs that are exempt from pediatric study requirements
  - Last year, 58% of all drugs approved by FDA's CDER were designated as orphan drugs and exempt from PREA





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

FDA Report to Congress: Orphan Drugs and PREA

- In August, FDA released a report titled **Pediatric Labeling of Orphan Drug**s.
- The study examined all FDA-approved orphan drugs that treat rare conditions that occur in children as well as adults.
- **36 %** of approved orphan indications that were relevant to pediatrics were missing important pediatric use information, either having no pediatric information at all or missing some information.
  - Of those 127 indications relevant to the pediatric population but lacking pediatric data, 81 contain no information at all related to the efficacy, safety, dosing, and formulation of the drug for children.

FDA Report to Congress: Orphan Drugs

- The law allows FDA to apply PREA to orphan drugs through rulemaking
- **FDA should act quickly to allow to remove the PREA orphan exemption**

(k) RELATION TO ORPHAN DRUGS.—

(1) IN GENERAL; EXEMPTION FOR ORPHAN INDICATIONS.—Unless the Secretary requires otherwise by regulation and except as provided in paragraph (2), this section does not apply to any drug or biological product for an indication for which orphan designation has been granted under section 526.

Section 505B(k) of the Federal Food, Drug and Cosmetic Act

Pediatric Cancer

- The FDA Reauthorization Act of 2017 made important changes to BPCA and PREA.
  - Allows FDA to require pediatric studies of cancer drugs based on molecular target, rather than being limited to when the cancer indication is identical in children and adults
- Going forward, we'd like to see the law move towards a **disease agnostic approach**, allowing children who experience other conditions to benefit from promising therapies that may have a different indication in adults.

Non-Compliance

- FDA has issued **31 PREA non-compliance letters** since 2013
  - **8** of these sponsors have fulfilled or been released from their PREA requirements
  - **23** of the sponsors have not completed their PREA-required studies or been released from the requirement to conduct those studies.
    - **6** of these requests have been pending since 2013.
- FDA needs additional **enforcement tools** to ensure that critical pediatric studies are completed in a timely manner.

Transparency

- There should be **greater transparency** around BPCA studies in order to improve collaboration and coordination between industry, researchers, and patients.
- Details on BPCA studies are not made public until after all the studies are completed, which can be about 5-10 years after they are requested by FDA.
- FDA can't share specifics of BPCA study requests with counterparts in other countries, which hinders their ability to perform pediatric studies in those countries.
- The public is not informed when companies decline BPCA study requests.

Ongoing Challenges

- Many therapeutics are not manufactured in **formulations** that are easily administrable to children.
  - Many drugs have not been appropriately studied for routes of administration common in children.
- Studies for **neonates** continue to lag behind those for other children.
  - Progress has been made with the creation of a permanent position for a neonatologist in the Office of Pediatric Therapeutics and the recent release of guidance on pharmacology for neonatal studies, but more must be done.
- Children from some **racial/ethnic groups** are often not adequately represented in clinical trials.
  - The study population for a therapy used to treat a given condition must be reflective of the population afflicted with the condition.

Thank You

- Tremendous progress has been made in recent years to ensure that drugs are safe and effective for children.
  - Without the support of FDA, none of this would be possible.
- We look forward to working with FDA to continue making progress for children.