Pediatric Research Equity Act (PREA)

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Disclosure Statement

- I have no financial relationships to disclose relating to this presentation

- The views expressed in this talk represent my opinions and do not necessarily represent the views of FDA

Outline

Pediatric Research Equity Act (PREA)

General Principles
- Description of the Statute
- Study Requirements
- Basis for Waivers and Deferrals

Impact on Non-Prescription Products

Unique study requirements
- Determination of safe use

Pediatric Drug Development - General Principles

ICH E11 (R1) Guideline

- Ideally, products should have a pediatric assessment at the same time as adults when appropriate
- Promote early planning by drug developers regarding investigational product development in all pediatric age groups
- Ensures products anticipated to have public health benefits in the pediatric population are studied in all relevant age groups
- Applies to both new drug applications (NDAs) and biologics license applications (BLA)
- PREA does not apply to monograph active ingredients

When PREA Requirements Apply

- Pediatric study requirements under PREA apply whenever the product being developed contains any of the features:
  - ➢ New active ingredient
  - ➢ New indication
  - ➢ New dosage form
  - ➢ New dosing regimen
  - ➢ New route of administration
  - ➢ Molecularly targeted cancer drugs

Criteria for PREA Waivers

- Sponsors may request full (all pediatric ages) or partial (subset of pediatric population) waiver of required assessments:

1. Necessary studies are impossible or highly impracticable; OR
2. Evidence strongly suggests the drug/biologic would be ineffective or unsafe; OR
3. Drug/biologic does not represent a meaningful therapeutic benefit over existing therapies for pediatric patients AND is not likely to be used by a substantial number of pediatric patients; OR
4. Reasonable attempts to produce a pediatric formulation necessary for that age group have failed (partial waiver only)

505B(a)(4) of the FD&C Act

Applying PREA to Non-Prescription Analgesic/Antipyretic Drug Development

FDA has authority to require studies in the pediatric population for new analgesic/antipyretic products developed in adults for OTC use that are subject to PREA

FDA must determine, if any of the waiver criteria would apply to a new product developed for adult use

- By determining if the new analgesic product would be unsafe or ineffective in the pediatric population or a subset of the population
- If it would be feasible to study the product in the pediatric population or a subset of the population
- If the product would provide a meaningful therapeutic benefit over existing therapies AND be used by a substantial number of pediatric patients

PREA Required Assessment

As with prescription programs, nonprescription drug developers must submit an Initial Pediatric Study Plan (iPSP) outlining how they plan to support safety and effectiveness of their drug in pediatric patients.

May submit assessments at time of the NDA submission or deferred studies, in the form of PREA post marketing requirements (PMR) are issued to be submitted at a pre-specified time after the approval of the NDA.

Specific assessments may include:

- Efficacy studies
- Pk and dose finding studies
- Safety studies which may include:
- Consumer use studies I.e., label comprehension, actual use, self-selection studies

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