PDUFA VI Supports the Advancement of Medicines for Pediatric Patients

For 25 years, the Prescription Drug User Fee Act (PDUFA) has provided FDA with stable, predictable funding that has resulted in significantly shortened drug review times, greater consistency and increased predictability, while preserving FDA's "gold standard" for safety and effectiveness.

Timely reauthorization of PDUFA is essential to support FDA's mission of protecting public health and promoting innovation for patients, particularly in areas where there is tremendous unmet need and unparalleled scientific complexity.

OVERCOMING CHALLENGES

Developing medicines for pediatric diseases poses unique scientific, ethical and operational challenges.

- Many pediatric diseases have small patient populations, making it challenging to recruit sufficient numbers of patients into clinical trials.
- Children often react differently to a drug than adult patients, and a disease may progress differently.
- Children may also require unique formulations and dosing for accurate and safe delivery of medicines (e.g., liquid or chewable tablets).

Despite these challenges, biopharmaceutical research companies remain committed to the development of innovative therapies and cures for pediatric patients.

PDUFA VI: SPURRING INNOVATION THROUGH SCIENCE

PDUFA VI will help the FDA keep pace with the latest scientific and medical advances in order to accelerate pediatric research and advance innovative treatment options for patients with unmet needs. The PDUFA VI agreement will enhance both the development and regulatory review of new medicines through provisions that provide greater predictability and efficiency, equipping the FDA to:

Keep pace with the latest scientific and technological advances

- Facilitate use of innovative clinical trial designs, including adaptive designs and new modeling approaches, which are particularly impactful when there is a small patient population
- Advance the use of real-world evidence (RWE), including both safety and efficacy data, in regulatory decision-making
- Increase use of drug development tools, including biomarkers

Streamline and enhance regulatory review

- Increase resourcing and support for the Breakthrough Therapy Program
- Integration of Rare Disease Program staff into review teams in order to provide expertise
- Streamline review of combination products (products composed of a drug or biologic and a device)

Accelerate the integration of the patient perspective

- Use of patient-reported outcomes (PROs) and meaningful patient input in drug development and review