FDA's Efforts to Optimize Medical Device Innovation for Pediatrics

Few medical devices are indicated and labeled for a pediatric population. With increasing numbers of novel devices approved for adults, the number of devices approved for pediatrics has also increased. However, despite legislative and regulatory changes designed to incentivize pediatric device development, the percentage of novel devices approved for use in pediatric populations over the past decade has been relatively stagnant. The lack of devices designed, evaluated, and approved for pediatrics, not only limits access of pediatric patients to potentially beneficial novel devices, but also leads to off-label use of devices, potentially altering the benefit-risk profile. Some products are designed specifically for children, while others are borrowed from adult applications or produced for more general use. Designing pediatric medical devices can be challenging: children are often smaller and more active than adults, body structures and functions change throughout childhood, and children may be long-term device users -- bringing new concerns about device longevity and long-term exposure to implanted materials.

FDA is committed to supporting the development and availability of safe and effective pediatric medical devices. Current initiatives include:

- Increasing the number of medical devices with labeling for pediatric patients by incorporating known information about device effects in other populations to support pediatric indications.
- Recruiting pediatric experts for FDA advisory panels whenever there is a reasonable likelihood that the device under discussion will be used for children.
- Collecting data on the unmet needs for pediatric medical devices and the barriers to the development of new pediatric devices.
- Protecting children who participate in clinical trials.

Programs that Foster Pediatric Medical Device Development

To encourage device innovation for medical conditions that impact small populations, including pediatric populations, the Federal Food, Drug, and Cosmetic Act (FD&C Act)includes the Humanitarian Use Device (HUD)/Humanitarian Device Exemption (HDE) pathway. The program provides a marketing pathway for devices intended to benefit patients in the treatment or diagnosis of a disease or condition that affects or is manifested in not more than 8,000 individuals in the US per year. Since 2008, the year Congress began requiring annual Pediatric Medical Device Reports, 29 devices have been approved through the HUD/HDE pathway. Seven of those have a pediatric indication, and 4 are indicated only in pediatric populations.

In 2007, with enactment of the Pediatric Medical Device Safety and Improvement Act (PMDSIA), the Pediatric Device Consortia (PDC) Grant Program was created. The program facilitates the development, production, and distribution of pediatric medical devices through funding of nonprofit consortia. The consortia are responsible for mentoring, supporting, and providing regulatory and marketing consultation to pediatric medical device developers. Nineteen pediatric medical devices supported by this program have been cleared to date.

FDA's work to support pediatric medical device development continues through authorizations granted under the Food and Drug Administration Reauthorization Act (FDARA). Specifically, the FDA must convene a public meeting to discuss ways to:

- improve research infrastructure and research networks to facilitate the conduct of clinical studies of pediatric devices;
- appropriately use extrapolation under section 515A(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360e-1(b));

- enhance the appropriate use of postmarket registries and data to increase pediatric medical device labeling;
- increase FDA assistance to medical device manufacturers in developing devices for pediatric populations that are approved or cleared, and labeled, for their use;
- identify current barriers to pediatric device development and incentives to address such barriers

FDA's Proposed Approach to Encourage Pediatric Medical Device Development

To address the pediatric needs identified in FDARA, FDA is focusing on integrating three fundamental areas: optimizing evidence generation, creating regulatory value and simplicity, and developing a supportive marketplace.

Considering the most efficient and least burdensome methods of generating clinical evidence is especially important for pediatric medical device development due to the unique issues in this field. FDA's Center for Devices and Radiological Health (CDRH) is leading efforts, including the creation of policies and procedures, to streamline clinical trial design and the evidence generation enterprise across the total product lifecycle of devices for all devices, including those for pediatric populations.

Collaborating with industry on the National Evaluation System for health Technology (NEST), and incorporating Real-World Evidence (RWE) generation strategies, the Agency is developing a more efficient and balanced approach toward pre- and post-market data collection, in concert with our patient-centered benefit-risk approach. Through collaborative efforts, FDA is identifying opportunities for developing scalable efficiencies within this framework, specific to pediatric device development, and consideration of novel evidence generation opportunities offering solutions to barriers in pediatric medical device development.

The FDA is dedicated to facilitating innovation and supporting opportunities to motivate industry to enter, sustain, and innovate in the pediatric medical device market. We can learn from the similarities and differences between the drug and device markets and the incentives that compelled development of the pediatric drug market Financial strategies for small and large device companies to address economic challenges in the pediatric market should be considered along with current collaborative and unique business models for supporting pediatric device development.

The FDA continues to promote collaboration and dialogue with stakeholders in the development of pediatric medical devices. By working together to foster a system that supports technology innovation to serve the complex needs of children and special populations, we will accelerate device development for all Americans.