U.S. Regulatory Initiatives to Promote Pediatric Medical Device Development

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Disclosures

- Nothing to disclose

Pathways to U.S. Patients

- Investigational Device Exemption (IDE)
  - Clinical trials for significant risk devices
  - Studies can be for research/publication or to support marketing approval or labeling claims
  - Traditional EFS, Feasibility, Pivotal or Sponsor - Investigator

- Marketing applications
  - 510(k): premarket notification
  - De Novo
  - PMA: premarket approval application
  - HDE: humanitarian device exemption

Pediatric Population at CDRH

- Pediatric
  - Patients ≤ 21 years at the time of the diagnosis or treatment
- Pediatric "subgroups"
  - Neonates (birth – 1 month)
  - Infants (>1 month – 2 yrs)
  - Children (>2 yrs – 12 yrs)
  - Adolescents (>12yrs – 21 yrs)
- Defined in *Premarket Assessment of Pediatric Medical Devices Guidance*, available at:

  http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm089740.htm

Breakthrough Device

- Intended for medical devices that provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions
- More information at: https://www.fda.gov/medical-devices/how-study-and-market-your-device/breakthrough-devices-program

TPLC Advisory Program Pilot

- Intended to increase access to high quality, safe and innovative medical devices
- De-risk the medical device valley of death by providing industry with earlier and more frequent interactions with CDRH
- More information at: https://www.fda.gov/medical-devices/medical-devices-news-and-events/cdrh-launches-total-product-life-cycle-advisory-program-pilot

Safer Technologies Program

- Intended for devices that are reasonably expected to significantly improve the safety of currently available treatments
- Target an underlying disease or condition associated with morbidities and mortalities less serious than those eligible for the Breakthrough Devices Program
- More information at: https://www.fda.gov/medical-devices/how-study-and-market-your-device/safer-technologies-program-step-medical-devices

Pre-submissions

- Obtain FDA feedback
  - Before IDE or marketing submission
  - **Before initiating non-clinical studies**
  - Before examining registry data
- Written feedback and/or Meeting (virtual or hybrid)
- Feedback on regulatory pathway or proposed clinical/nonclinical data plans
- More information at: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/requests-feedback-and-meetings-medical-device-submissions-q-submission-program

Registries

- Use of registries to transition from off-label use in a specific patient population/disease state to on-label use
  - Example: CPB devices to FDA cleared ECMO devices
- Use of databases to prospectively create control populations or construct performance goals
- Prospective clinical trials embedded in registries
  - Example: Berlin Heart Active Driver in the ACTION Registry
- Track long-term device performance

If using a registry for these purposes,
it is recommended that you discuss with FDA first.

Example

First Pediatric Cardiac Device IDE Trial (Berlin Heart EXCOR® Active Driver) has Completed Enrollment Using the ACTION Registry

August 11, 2023

40 Pediatric Patients Enrolled Over 10 Months at 15 ACTION Sites.

Leveraging Existing Data

- Leveraging relevant available clinical data to support pediatric or adult claims in PMAs and HDEs

- May be appropriate to extrapolate:
  - Course of the disease and effects of the device similar in adults and pediatric patients
  - Endpoints relevant to the new population

- *Leveraging Existing Clinical Data for Extrapolation to Pediatric Uses of Medical Devices Guidance*, http://www.fda.gov/downloads/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm444591.pdf

Example

FDA APPROVES LABELING UPDATE FOR ABBOTT'S HEARTMATE 3 HEART PUMP FOR USE IN PEDIATRIC PATIENTS

- Abbott's HeartMate 3™ heart pump approved for use for pediatric patients battling advanced heart failure

- This life-saving technology provides new treatment option for underserved population

(1)

ABBOTT PARK, Ill., Dec. 17, 2020 /PRNewswire/ -- Abbott (NYSE: ABT) today announced the U.S. Food and Drug Administration (FDA) has approved updated

Summary

- There are multiple pathways to incentivize devices in pediatric populations.
- Together, we can help get appropriate devices on label for pediatrics
- When considering the pediatric population, FDA recommends discussing the project early with FDA to ensure alignment

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