DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2019-N-4041]

Advancing the Development of Pediatric Therapeutics: Pediatric Clinical Trial Endpoints for Rare Diseases with a Focus on Pediatric Patient Perspectives; Public Workshop

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop.

SUMMARY: The Office of Pediatric Therapeutics, Food and Drug Administration (FDA), is announcing a public workshop entitled “Advancing the Development of Pediatric Therapeutics (ADEPT 6): Pediatric Clinical Trial Endpoints for Rare Diseases with a Focus on Pediatric Patient Perspectives.” The purpose of this workshop is to discuss pediatric patient-specific engagement in the development of clinical trial endpoints for rare diseases.

DATES: The public workshop will be held on November 12, 2019, from 8 a.m. to 4:30 p.m. See the SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public workshop will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, the Great Room (Rm. 1503-A), Silver Spring, MD 20993-0002. Entrance for the public workshop participants (non-FDA employees) is through Building 1, where routine security check procedures will be performed. For parking and security information, please refer to https://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm.
Patient engagement is critical in the development of patient-focused study endpoints that measure clinical benefit in clinical trials. Asking patients what aspects of their disease they consider important to measure is especially important for rare diseases, given the lack of established endpoints for many rare diseases, the small number of patients available for enrollment in trials, and the heterogeneity of disease manifestations (e.g., between patients and over time). While there is increased emphasis on incorporating the patient voice in rare disease drug development activities, there is an increased need for pediatric patient-specific engagement efforts. Pediatric rare disease drug development would benefit from direct and early involvement of pediatric patients and their caregivers in determining the most relevant and clinically meaningful endpoints and outcome assessment tools for use in clinical trials.

II. Topics for Discussion at the Public Workshop

In this workshop, FDA will obtain the pediatric patient perspective on their disease/condition and what is most important to consider when designing rare disease trials. There will also be discussion regarding patients’ thoughts on clinical endpoints that are currently being used in clinical trials, potential areas of innovation, and how to create processes that might include pediatric patients and their caregivers as collaborators in endpoint development in early
stages of medical product development (e.g., protocol design). The morning session will focus on identifying endpoints that capture important aspects of how pediatric patients feel and function. The afternoon session will focus on steps for development of clinical outcome assessment tools for use in pediatric patient populations and the potential role of child and youth friendly technology in endpoint assessments.

III. Participation in the Public Workshop

Registration: Persons interested in attending this public workshop must register online at: https://www.eventbrite.com/e/adept-6-workshop-pediatric-clinical-trial-endpoints-for-rare-diseases-registration-67523118465 by November 5, 2019. For those without internet access, please contact Terrie Crescenzi or Elizabeth Sanford (see FOR FURTHER INFORMATION CONTACT) to register.

Registration is free and based on space availability, with priority given to early registrants. Onsite registration on the day of the meeting will be based on space availability. Registration information, the agenda, and additional background materials can be found at http://wcms-internet.fda.gov/news-events/fda-meetings-conferences-and-workshops/advancing-development-pediatric-therapeutics-adept-6-pediatric-clinical-trial-endpoints-rare.

If you need special accommodations due to a disability, please contact Elizabeth Sanford (see FOR FURTHER INFORMATION CONTACT) at least 7 days in advance. Persons attending the meeting are advised that FDA is not responsible for providing access to electrical outlets.

Streaming Webcast of the Public Workshop: This public workshop will also be webcast. Login URL: https://collaboration.fda.gov/adept6/
If you have never attended a Connect Pro event before, test your connection at https://collaboration.fda.gov/common/help/en/support/meeting_test.htm. To get a quick overview of the Connect Pro program, visit https://www.adobe.com/go/connectpro_overview.

FDA has verified the website addresses in this document, as of the date this document publishes in the Federal Register, but websites are subject to change over time.

Transcripts: Please be advised that as soon as a transcript of the public workshop is available, FDA will post it at http://wcms-internet.fda.gov/news-events/fda-meetings-conferences-and-workshops/advancing-development-pediatric-therapeutics-adept-6-pediatric-clinical-trial-endpoints-rare

Dated: October 4, 2019.

Lowell J. Schiller,

Principal Associate Commissioner for Policy.

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