FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)

Meeting of the Pediatric Oncology Subcommittee of the Oncologic Drugs Advisory Committee (pedsODAC)
May 11-12, 2021

DRAFT QUESTIONS

Day 1: May 11, 2021

Discussion Issues Relating to the Development of Pediatric Patient-Reported Outcomes Version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE)

1. **DISCUSSION:** Consider how patient self-report by children of symptoms attributable to a drug in a clinical trial might inform patients, parents, and providers about its tolerability and decisions regarding use.

2. **DISCUSSION:** Since only children greater than 7 years of age are able to reliably self-report symptoms, discuss the role for supplementing experiences of younger children using care-giver reports of "observable" symptoms (frequency, severity, and interference)?

3. **DISCUSSION:** Consider the logistical and operational challenges to collecting and analysis of data from patient self-report of symptoms.

4. **DISCUSSION:** Consider how best the constellation of self-reported symptoms in children and adolescents should be selected to be used in extending FDA's Project Patient Voice to children.

5. **DISCUSSION:** Consider whether data obtained in real time from children's self-report of symptomatic adverse events (AEs) could possibly impact the conduct of a clinical trial or inform an individual study participant's clinical management. Consider specific assessment and reporting requirements to be included in the protocol.

6. **DISCUSSION:** Consider how pediatric PRO-CTCAE might contribute to planning and implementation of supportive care and survivorship research strategies in children.

DRAFT QUESTIONS (cont.)

Day 2: May 12, 2021

Discussion of Real-world evidence (RWE) for regulatory use in pediatrics, real-world data (RWD) resources, and RWD and RWE to advance pediatric safety assessments of oncology drug products in children within the context of the FDA Framework for RWE

1. **DISCUSSION:** Consider the potential of existing and future RWD resources that may provide RWE to support pediatric cancer drug development programs. Consider potential uses to inform regulatory decision-making. Consider specific pediatric cancer drug development programs that might benefit.

2. **DISCUSSION:** Given the discussion of the FDA framework on the use of RWD and RWE on regulatory decision-making, consider how best to assess the appropriateness of existing or emerging data sources as potential sources of RWD. Discuss critical attributes of such data.

3. **DISCUSSION:** Consider the real and perceived limitations to RWE from existing and developing registries in pediatric cancer drug development as a result of the General Patient Data Regulations (GPDR) in the European Union.

4. **DISCUSSION:** Consider possible mechanisms for how and by whom attribution of RWD/RWE- generated adverse events (AEs) of new cancer drugs can be accomplished, and data optimally aggregated to inform patients and providers.