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

Pediatric Oncology Subcommittee of the Oncologic Drugs Advisory Committee (pedsODAC) Meeting
May 11-12, 2022

DRAFT QUESTIONS

Day 1: May 11, 2022

Topics Related to Considerations for Evaluating Planned Waivers of Pediatric Investigations of Same in Class Agents

1. **DISCUSSION:** Consider the degree of unmet clinical need in a specific disease context that should influence decisions related to planned waiver requests for pediatric studies of multiple same-in-class novel agents.

2. **DISCUSSION:** Consider the importance of any comparative efficacy results of same in class agents in one or more adult cancers as well as comparative toxicity data (type, magnitude, and frequency) that could contribute to a decision where evaluation of more than one same in class product in children might be warranted.

3. **DISCUSSION:** Consider whether differences in specific product quality indicators, dosage forms, route of administration, impact clinical benefit considerations and influence a decision to investigate multiple same in class products.

4. **DISCUSSION:** Consider the importance of non-clinical efficacy data on whether pediatric investigations of more than one same-in-class products are warranted in children and if/when pre-clinical studies in pediatric-specific models might be required.

5. **DISCUSSION:** Consider the specific pharmacological parameters that should be considered and the importance of central nervous system (CNS) penetration when primary CNS tumors may be key target tumors of interest when evaluating the need for pediatric investigation of more than one same in class agent.

6. **DISCUSSION:** Discuss the extent to which sponsors should include sufficient data to address the features discussed in initial Pediatric Study Plans (iPSPs) to inform assessment and decision-making and whether other features should be considered in decision-making about waiving requirements to investigate multiple same in class drugs.

DRAFT QUESTIONS (cont.)

Day 2: May 12, 2022

Topics Relating to the End of Induction Response in High-Risk Neuroblastoma

1. **DISCUSSION:** Please discuss the potential benefits and limitations to using an intermediate clinical endpoint in the evaluation of new drugs under development for the first-line treatment of patients with high-risk neuroblastoma.

2. **DISCUSSION:** Please discuss the strength of the evidence for using end-of-induction response as a prognostic factor and to assess antitumor activity of investigational treatments during the induction phase of treatment.

3. **DISCUSSION:** Please discuss how end-of-induction response is used in clinical decision-making and the implications of its use in the design and conduct of clinical trials investigating new treatments for patients with high-risk neuroblastoma.

4. **DISCUSSION:** Given the current strength of evidence for using response at the end-of-induction to predict patient outcome and assess antitumor activity, consider the appropriate use of this endpoint in clinical trials.

5. **DISCUSSION:** If there is sufficient evidence to support future efforts, please provide recommendations regarding interest, feasibility and future steps to validation of end-of-induction response as a clinical endpoint in the first-line treatment of patients with high-risk neuroblastoma.