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

Pediatric Subcommittee of the Oncologic Drugs Advisory Committee (pedsODAC) Meeting
FDA White Oak Campus, Building 31 Conference Center, the Great Room (Rm. 1503)
10903 New Hampshire Avenue, Silver Spring, Maryland
June 20, 2019

QUESTIONS

AM Session

Issues Relating to the Development of the Targets Associated with Specific Cell Lineage Determinants List

1. **DISCUSSION:** Please discuss any new or emerging data that provide sufficient evidence for the addition of a molecular target to the List of Molecular Targets Associated with Specific Cell Lineage Determinants.

2. **DISCUSSION:** Please discuss any new or emerging data that provide sufficient evidence that a relevant target currently on this list should be removed.

Issues Relating to the Development of the Targets on Normal Immune Cells and Cells in the Tumor Microenvironment List

3. **DISCUSSION:** Please discuss any new or emerging data that provide sufficient evidence for the addition of a molecular target to the List of Relevant Targets on Normal Immune Cells and Cells in the Tumor Microenvironment.

4. **DISCUSSION:** Please discuss any new or emerging data that provide sufficient evidence for the deletion of a target on this list.

5. **DISCUSSION:** Please discuss specific recommendations for how best to evaluate and/or prioritize combinatorial approaches to evaluating agents directed at targets on normal immune cells.

PM Session

1. **DISCUSSION:** Given the mechanism of action of ONC201 and broad antitumor activity observed in a range of preclinical cancer models, please discuss possible options for evaluation of ONC201 in pediatric pre-clinical tumor models and possible pediatric development of ONC201 beyond high grade gliomas.

2. **DISCUSSION:** Please discuss the CNS penetration properties of ONC201 and any potential role in addressing brain metastases in children.

3. **DISCUSSION:** Please consider the plans for administering ONC201 in combination with other treatments such as radiation therapy, targeted therapies or chemotherapy regimens and recommendations for isolating the effect of ONC201.

QUESTIONS (cont.)

4. **DISCUSSION:** Please address any potential short-term or long-term toxicity unique to the pediatric population that might justify exclusion of any pediatric age groups not planned for study (e.g., patients younger than 2 years of age are ineligible in ongoing Study ONC014).

5. **DISCUSSION:** Please comment on the potential endpoints that could be used in future clinical trials designed to evaluate the isolated efficacy of ONC201 in pediatric patients.