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

Pulmonary-Allergy Drugs Advisory Committee (PADAC) Meeting
FDA White Oak Campus, Building 31 Conference Center, the Great Room (Rm. 1503)
10903 New Hampshire Avenue, Silver Spring, Maryland
May 8, 2019

DRAFT QUESTIONS

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1. **DISCUSSION:** Discuss the efficacy of dry powder mannitol (DPM) for the proposed indication of the management of cystic fibrosis to improve pulmonary function in patients 18 years of age and older in conjunction with standard therapies. Include the following topics in your discussion:
    a. Effect on FEV1, including effect size and durability of effect
    b. Secondary endpoints, particularly exacerbations and the Cystic Fibrosis Questionnaire – Revised respiratory domain score
    c. Statistical persuasiveness

2. **DISCUSSION**: Discuss the safety data for DPM for the proposed use in patients with cystic fibrosis 18 years of age and older, particularly exacerbation and hemoptysis.

3. **VOTE:** Do the data provide substantial evidence of efficacy for DPM for the proposed indication of the management of cystic fibrosis to improve pulmonary function in patients 18 years of age and older in conjunction with standard therapies?
    a. If no, what further data are needed?

4. **VOTE**: Are the safety data adequate to support approval of DPM for the proposed indication of the management of cystic fibrosis to improve pulmonary function in patients 18 years of age and older in conjunction with standard therapies?
    a. If no, what further data are needed?

5. **VOTE**: Is the benefit-risk profile adequate to support approval of DPM for the proposed indication of the management of cystic fibrosis to improve pulmonary function in patients 18 years of age and older in conjunction with standard therapies?
    a. If no, what further data are needed