



Inborn Errors of Metabolism Public Meeting on Patient-Focused Drug Development



June 10, 2014

8:00 – 9:00 am	Registration
9:00 – 9:10 am	Welcome and Opening Remarks Sara Eggers, PhD <i>Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER), FDA</i> Donna Griebel, MD <i>Director, Division of Gastroenterology and Inborn Error Products (DGIEP), CDER, FDA</i>
9:10 – 9:30 am	Background and Context Theresa Mullin, PhD <i>Director, OSP, CDER, FDA</i> Teresa Buracchio, MD <i>Medical Officer, DGIEP, CDER, FDA</i> Sara Eggers, PhD <i>OSP, CDER, FDA</i>
9:30 – 10:00 am	Panel #1 Comments on Topic 1 Topic 1: Neurological manifestations of inborn errors of metabolism that matter most to patients. A panel of patients and patient representatives will provide comments to start the discussion.
10:00 – 10:45 am	Large-Group Facilitated Discussion on Topic 1 Patients and patient representatives in the audience are invited to add to the dialogue.
10:45 – 11:00 am	Break
11:00 – 11:30 am	Panel #2 Comments on Topic 2 Topic 2: Approaches to treating the neurological manifestations of inborn errors of metabolism and perspectives on informed consent for clinical trials.
11:30 am – 12:15 pm	Large-Group Facilitated Discussion on Topic 2
12:15 – 12:45 pm	Open Public Comment
12:45 – 1:00 pm	Closing Remarks Teresa Buracchio, MD <i>CDER, FDA</i>

Discussion Questions

Topic 1: Disease Signs, Symptoms, and Daily Impacts That Matter Most to Patients

1. Of all the signs or symptoms that you/your child experiences because of the condition, which 1-3 neurologic/neuropsychological signs and/or symptoms have the most significant impact on your/your child's life? (Examples may include seizures, decreased muscle tone, sensory issues, etc.)
2. Are there specific activities that are important to you/your child but that you/your child cannot do because of these neurologic/neuropsychological signs or symptoms? (Examples of activities may include sleeping through the night, daily hygiene, going up the stairs, etc.)
3. How have your/your child's neurologic/neuropsychological signs or symptoms changed over time?

Topic 2: Patient Perspectives on Current Approaches to Treating Neurologic Manifestations of Inborn Errors of Metabolism and Informed Consent for Clinical Trials

1. What are you/your child currently doing to help treat the condition or its signs/symptoms? (Examples may include prescription medicines, herbal therapies, acupuncture, over-the-counter products, and other therapies including nondrug therapies such as diet modification.)
 - a. How well does this current treatment regimen treat the neurological symptoms of your/your child's disease? For example, how well do the treatments improve your/your child's ability to do specific activities?
2. Assuming there is no complete cure for your/your child's condition, what specific attributes would you look for in an ideal treatment for the condition?
3. In the informed consent process, what are important considerations to take into account in cases when the potential participant is a child? For example, how should the informed consent clearly communicate to the patient the potential benefits and risks of a study?

Docket Information

We encourage you to submit your written comments to the docket by August 11, 2014:

<http://www.regulations.gov/#!documentDetail;D=FDA-2014-N-0396-0001> or go to www.regulations.gov and search for: **inborn errors of metabolism patient-focused drug development**.