Inborn Errors of Metabolism Patient Meeting To Focus on Neurological Manifestations, Consent Issues for Children

The Food & Drug Administration’s June 10 half-day meeting with the patient community for diseases of inborn errors of metabolism is likely to bring together FDA review teams from two specialties: the Gastroenterology & Inborn Errors Products Division and the Division of Neurology Products.

The Gastroenterology Division is the lead group in this area. However, the focus on the neurological manifestations of the inborn errors suggests that the agency will also bring in expertise from the other review division. If that occurs, this will be one of the first of the ongoing patient group meetings to deal directly with the issue of shared cross-division expertise in reviews of products for conditions with unmet needs.

The half-day meeting (9 am to 1 pm) will be the eighth of 20 meetings part of the PDUFA V initiative intended to better understand patients’ perspective on drug development for certain diseases.

The Gastroenterology & Inborn Errors Division reviews products for such diseases as Fabry’s disease, Gaucher’s disease and ulcerative colitis. The division is headed up by Donna Griebel.

The neurology division has already held a patient-focused meeting in its review area, convening a session on narcolepsy in September last year. (See our September 25 note, “Narcolepsy Patient Meeting: First Rare Disease PDUFA Session Plays to Over-Subscribed Meeting/Webcast.”)

A panel of patients will be asked to engage in dialogue to answer questions on the symptoms with significant daily impact on patients, with particular focus on neurologic impact.

FDA will also be looking for information on the current treatments to manage neurological symptoms (which might include seizures, decreased muscle tone, and sensory issues).

Similar to the discussion a year earlier with HIV patients (on June 14, 2013), FDA is interested in the issues of informed consent in trials for potential products for inborn errors. There is a major difference in the focus on informed consent. In the case of the HIV patients, the issue was giving up access to useful chronic treatments in the search for potential cures. (See our June 18, 2013 note “HIV Cure Research: Further Reflections From June 14 FDA Patient Feedback Meeting.”)

For inborn errors of metabolism research, the consent issues relate to parents participating in making choices for children and to the child being asked for “assent” to participate in the trial.
FDA will be asking for advice on the shared decision-making responsibility. One of the questions for the meeting asks: “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?”

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FDA Pre-Meeting Questions for June 10 Session with Patients on Inborn Errors of Metabolism

**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**

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.) 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. The process of informed consent is an important way for researchers to communicate the purpose of a clinical trial and the potential benefits and risks of the trial so that people can make an informed decision about whether to participate. Informed consent also ensures that parents are fully informed and are given opportunities to ask questions about the clinical trial. In addition to informed consent from parents, assent from children may also be needed. Assent is the term used to describe when a child agrees to be in a clinical trial. Among other considerations, children should be old enough to understand basic facts about the clinical trial in order to provide assent to participate. 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?