Sickle-Cell Disease Public Meeting on Patient-Focused Drug Development

February 7, 2014

9:00 – 10:00 am  Registration

10:00 – 10:05 am  Welcome
   Sara Eggers, PhD
   Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER), FDA

10:05 – 10:10 am  Opening Remarks
   Ann Farrell, MD
   Director, Division of Hematology Products (DHP), CDER, FDA

10:10 – 10:20 am  Overview of FDA’s Patient-Focused Drug Development Initiative
   Theresa Mullin, PhD
   Director, OSP, CDER, FDA

10:20 – 10:35 am  Background on Sickle Cell Disease and Treatment
   Nicole Verdun, MD
   DHP, CDER, FDA

10:35 – 10:45 am  Overview of Discussion Format
   Sara Eggers, PhD
   OSP, CDER, FDA

10:45 – 11:40 am  Pediatric and Young Adult Perspective on Topic 1: The effects of sickle cell disease that matter most to patients
   A panel of caregivers and young adult patients will provide comments followed by a large-group facilitated discussion with participants in the audience.

11:40 – 12:30 pm  Adult (Age 23+) Perspective on Topic 1
   A panel of patients and patient representatives will provide comments followed by a large-group facilitated discussion with participants in the audience.

12:30 – 1:30 pm  Lunch

1:30 – 1:35 pm  Afternoon Welcome
   Sara Eggers, PhD
   OSP, CDER, FDA

1:35 – 2:05 pm  Panel Discussion on Topic 2: Patients’ perspectives on treatments for sickle cell disease
   A panel of pediatric and adult patients or patient representatives will provide comments to start the discussion.
Appendix: Discussion Questions

If commenting on behalf of a child or other loved one who has sickle cell disease, please answer the following questions as much as possible from the patient’s perspective.

**Topic 1: The effects of sickle cell disease that matter most to you**

1) Of all of the ways that sickle cell disease affects your health, which 1-3 effects have the greatest impact on your life? (Examples may include pain crises, breathing problems, difficulty concentrating, tiredness, infections, and others.)

2) How does sickle cell disease affect your life on an “average” day?
   a) Are there activities that you cannot do at all or as well as you would like? Please describe, using specific examples. (Examples may include sleeping through the night, concentrating at work or at school, participating in physical activities, and others.)

3) How does sickle cell disease affect your life on the “worst” days, such as days when you have a pain crisis or have to be hospitalized for some reason?
   a) Are there activities that you cannot do at all or as well as you would like? Please describe, using specific examples.

4) What worries you most about how sickle cell disease could affect your health in the future?

5) What specific concerns do you have about sickle cell disease:
   a) In infants and young children?
   b) In adolescents and young adults?
   c) In older adults?
Topic 2: Perspectives on treatments for sickle cell disease

1) Are you currently using any prescription medicines or medical treatments to prevent or treat any negative effects of your sickle cell disease? Please describe these treatments, which may include blood transfusions, supplemental oxygen and prescription medications such as hydroxyurea, antibiotics, pain medications, and others.

   a) How well do these treatments work for you? For example, how well do they reduce your number of pain crises, hospitalizations, or strokes? How well do they help you manage your pain, breathing difficulties, or other health effects?

   b) What are the biggest problems with these treatments? (Examples may include side effects of medicine, going to the hospital for treatment, frequent blood tests, etc.) How do these problems affect your daily life?

2) Besides prescription medications, what else do you do to prevent or treat any negative effects of your sickle cell disease? Please describe any medications purchased at a store without a prescription, home remedies, diet changes, massages, or other therapies.

   a) What specific parts of your sickle cell disease do these treatments address?

   b) How well do these treatments work for you?

   c) What are the biggest problems with these treatments?

3) What parts of your sickle cell disease do your current treatments not treat at all or not as well as you would like?

4) Assuming that there is no cure for sickle cell disease, what specific things would you look for in an ideal treatment?

5) If you had the opportunity to consider participating in a clinical trial studying experimental treatments for sickle cell disease, what things would you consider when deciding whether or not to participate? Examples may include how severe your sickle cell disease is, how well current treatments are working for you, your concern about serious risks, and other things.