Patient-Focused Drug Development for Alpha-1 Antitrypsin Deficiency: Public Meeting

AGENDA

September 29, 2015

8:00 – 9:00 a.m.	**Registration**
9:00 – 9:05 a.m.	**Welcome** Donna Lipscomb, Facilitator *Office of Communication, Outreach and Development* *Center for Biologics Evaluation and Research (CBER), FDA*
9:05 – 9:10 am	**Opening Remarks** Ginette Michaud, M.D. *Deputy Director, Office of Blood Research and Review (OBRR)* *CBER, FDA*
9:10 – 9:20 a.m.	**Overview of FDA's Patient-Focused Drug Development Initiative** Pujita Vaidya, M.P.H *Office of Strategic Programs* *Center for Drug Evaluation and Research (CDER), FDA*
9:20 – 9:40 a.m.	**Background on Alpha-1 Antitrypsin Deficiency** L. Ross Pierce, M.D. *Medical Officer, Division of Hematology Clinical Review* *OBRR, CBER, FDA*
9:40 – 10:00 a.m.	**Overview of Discussion Format** Donna Lipscomb *CBER, FDA*
	Topic 1: The effects of Alpha-1 Antitrypsin Deficiency that matter most to you
10:00 – 10:30 a.m.	**Panel Discussion on Topic I** A panel of patients and caregivers will provide comments followed by a large-group facilitated discussion with participants in the audience.
10:30 -10:35 a.m.	**Presentation of Survey Data from the Alpha-1 Foundation** Elizabeth Johnson, *Alpha-1 Foundation*

10:35 – 11:30 a.m.	**Large-Group Facilitated Discussion: Topic 1** Patients and patient representatives in the audience will be invited to contribute to the discussion.
11:30 – 12:30 p.m.	**Lunch**
12:30 – 12:35 p.m.	**Afternoon Welcome** Donna Lipscomb *CBER, FDA*
	Topic 2: Patients' perspectives on current approaches to treatments
12:35 – 1:05 p.m.	**Panel Discussion on Topic 2** A panel of patients and caregivers will provide comments followed by a large-group facilitated discussion with participants in the audience.
1:05 – 1:10 p.m.	**Presentation of Survey Data from the Alpha-1 Foundation** Gordon Cadwgan, *Alpha-1 Foundation*
1:10 – 2:00 p.m.	**Large-Group Facilitated Discussion: Topic 2** Patients and patient representatives in the audience will be invited to contribute to the discussion.
	Topic 3: Patient perspectives on participating in a clinical trial to study experimental treatments
2:00 – 2:25 p.m.	**Large-Group Facilitated Discussion: Topic 3** Patients and patient representatives in the audiences will be invited to contribute to a discussion on Topic 2 Question 3.
2:25 – 2:30 p.m.	**Presentation of Survey Data from the Alpha-1 Foundation** John Walsh, *Alpha-1 Foundation*
2:30 – 3:00 p.m.	**Open Public Comment**
3:00 – 3:15 p.m.	**Closing Remarks** Ginette Michaud, M.D. *CBER, FDA*

Appendix: Discussion Questions

If commenting on behalf of a child or other loved one, please answer the following questions as much as possible from the patient's perspective.

Topic 1: The effects of Alpha-1 Antitrypsin Deficiency that matter most to you

1. Of all of the symptoms that you experience because of your condition, which one to three symptoms have the most significant impact on your life? (Examples may include:

   (a) For lung disease: shortness of breath during specific activities or at rest, chronic sputum, chronic cough, wheezing, weight loss, exacerbations of particular symptoms;

   (b) For liver disease: abdominal pain, loss of appetite, height & weight concerns.)

2. Are there specific activities that are important to you, but that you cannot do at all, or as well as you would like, because of your condition? Please describe, using specific examples. (Examples may include participating in physical activities, attending work/school, and family/social activities.)

3. How have your condition and its symptoms changed over time?

4. What worries you most about your condition?

Topic 2: Perspectives on current approaches to treatment

1. What are you currently doing to treat your condition or its symptoms? (Examples may include:
   (a) For lung disease: inhaled bronchodilators, inhaled corticosteroids, intravenous augmentation therapy with A_1-PI (Human) on a regular or intermittent basis;
   (b) Liver Disease: ursodiol).

   - How well do these treatments work for you?
   - What are the most significant disadvantages or complications of your current treatments, and how do they affect your daily life?
   - How has your treatment changed over time and why?
   - What aspects of your condition are not improved by your current treatment regimen?
   - What treatment has had the most positive impact on your life?

2. If you could create your ideal treatment, what would it do for you (i.e., what specific things would you look for in an ideal treatment)?

3. If you had the opportunity to consider participating in a clinical trial studying experimental treatments, what things would you consider when deciding whether or not to participate?