



Patient-Focused Drug Development Meeting Pulmonary Arterial Hypertension



May 13, 2014

12:00 – 1:00 pm	Registration
1:00 – 1:05 pm	Welcome Soujanya Giambone, MBA <i>Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER), FDA</i>
1:05 – 1:10 pm	Opening Remarks Ellis Unger, MD <i>Director, Office of Drug Evaluation I, CDER, FDA</i>
1:10 – 1:20 pm	Overview of FDA’s Patient-Focused Drug Development Initiative Theresa Mullin, PhD <i>Director, OSP, CDER, FDA</i>
1:20 – 1:30 pm	Background on Pulmonary Arterial Hypertension and Therapeutic Options Shari Targum, MD <i>Division of Cardiovascular and Renal Products (DCaRP), CDER, FDA</i>
1:30 – 1:40 pm	Overview of Discussion Format Soujanya Giambone, MBA <i>OSP, CDER, FDA</i>
1:40 – 2:10 pm	Panel #1 Comments on Topic 1 Topic 1: Disease symptoms and daily impacts that matter most to patients. A panel of patients and patient representatives will provide comments to start the discussion.
2:10 – 2:55 pm	Large-Group Facilitated Discussion on Topic 1 Patients and patient representatives in the audience are invited to add to the dialogue.
2:55 – 3:10 pm	Break
3:10 – 3:40 pm	Panel #2 Comments on Topic 2 Topic 2: Patient perspectives on current approaches to treating pulmonary arterial hypertension.
3:40 – 4:25 pm	Large-Group Facilitated Discussion: Topic 2
4:25 – 4:55 pm	Open Public Comment
4:55 – 5:00 pm	Closing Remarks Norman Stockbridge, MD, PhD <i>Director, DCaRP, CDER, FDA</i>

Discussion Questions

Topic 1: Disease symptoms and daily impacts that matter most to patients

- 1) Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life? (Examples may include chest pain, shortness of breath, difficulty concentrating, etc.)
- 2) Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition? (Examples of activities may include household chores, walking up the stairs, etc.)
 - a) How do your symptoms and their negative impacts affect your daily life on the best days?
On the worst days?
- 3) How has your condition and its symptoms changed over time?

Topic 2: Patient perspectives on current approaches to treating pulmonary arterial hypertension

- 1) What are you currently doing to help treat your condition or its symptoms? (Examples may include prescription medicines, over-the-counter products, and other therapies including non-drug therapies such as diet modification.)
 - a) How has your treatment regimen changed over time, and why?
 - b) How well does your current treatment regimen treat the most significant symptoms of your disease?
 - c) Have the medications for pulmonary arterial hypertension made a difference to you? If so, in what ways?
- 2) What are the most significant downsides to your current treatments, and how do they affect your daily life? (Examples of downsides may include bothersome side effects, going to the hospital for treatment, etc.)
- 3) Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?

Docket Information

We encourage you to submit your written comments to the docket by July 14, 2014:

<http://www.regulations.gov/#!documentDetail;D=FDA-2013-N-1041-0001> or go to

www.regulations.gov and search for: **pulmonary arterial hypertension patient-focused drug development.**