



Narcolepsy Public Meeting on Patient-Focused Drug Development



September 24, 2013

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 Narcolepsy and Therapeutic Options Ronald Farkas, MD, PhD <i>Lead Medical Officer, Division of Neurology Products, 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: Patients’ perspectives on disease symptoms and daily impacts that matter the most. 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: Patients’ perspectives on current approaches to treating narcolepsy
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 Eric Bastings, MD <i>Acting Director, Division of Neurology Products, 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 excessive daytime sleepiness, cataplexy, 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 sleeping through the night, work and school performance, etc.)
- 3) How have your symptoms changed over time?
 - a) Do your symptoms come and go? If so, do you know of anything that makes your symptoms better? Worse?

Topic 2: Patients' perspectives on current approaches to treating narcolepsy

- 1) What are you currently doing to help treat your condition or its symptoms? (Examples may include FDA-approved medicines, over-the-counter products, and other therapies including non-drug therapies such as lifestyle modifications.)
 - a) What specific symptoms do your therapies address?
 - b) How has your treatment regimen changed over time, and why?
- 2) How well does your current treatment regimen treat the most significant symptoms of your disease?
 - a) How well do these therapies improve your ability to do specific activities that are important to you in your daily life?
 - b) How well have these therapies worked for you as your condition has changed over time?
- 3) What are the most significant downsides to your current therapies, and how do they affect your daily life? (Examples of downsides may include bothersome side effects, inconvenient dosing schedules, access issues, etc.)
- 4) Assuming there is no complete cure for your condition, what specific things would you look for in an ideal therapy for your condition?