



# Huntington's Disease Public Meeting on Patient-Focused Drug Development



September 22, 2015

---

8:00 – 9:00 am	<b>Registration</b>
9:00 – 9:05 am	<b>Welcome</b> Soujanya S. Giambone, MBA <i>Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER), FDA</i>
9:05 – 9:10 am	<b>Opening Remarks</b> Billy Dunn, MD <i>Director, Division of Neurology Products (DNP), Office of New Drugs (OND), CDER, FDA</i>
9:10 – 9:20 am	<b>Overview of FDA's Patient-Focused Drug Development Initiative</b> Theresa Mullin, PhD <i>Director, OSP, CDER, FDA</i>
9:20 – 9:30 am	<b>Background on Disease Area and Treatment</b> Dave Podskalny, MD <i>Medical Officer, DNP, OND, CDER, FDA</i>
9:30 – 9:35 am	<b>Overview of Discussion Format</b> Soujanya S. Giambone, MBA <i>OSP, CDER, FDA</i>
9:35 – 10:05 am	<b>Panel #1 Comments on Topic 1</b> 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.
10:05 – 10:40 am	<b>Large-Group Facilitated Discussion on Topic 1</b> Patients and patient representatives in the audience are invited to add to the dialogue.
10:40 – 10:50 am	<b>Break</b>
10:50 – 11:20 am	<b>Panel #2 Comments on Topic 2</b> Topic 2: Patient perspectives on current approaches to treating Huntington's Disease. A panel of patients and patient representatives will provide comments to start the discussion.
11:20 – 11:55 pm	<b>Large-Group Facilitated Discussion on Topic 2</b> Patients and patient representatives in the audience are invited to add to the dialogue.
11:55 – 12:25 pm	<b>Open Public Comment</b>
12:25 – 12:30 pm	<b>Closing Remarks</b> Eric Bastings, MD <i>Deputy Director, DNP, OND, 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: ability to control movements, balance/coordination, difficulty concentrating, sleeping, mood/behavior 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 you need assistance because of your condition? (Examples of activities may include sleeping through the night, daily bathing/showering, cooking, eating, dressing, shopping etc.)
  - a) How do your symptoms affect your daily life on the **best days**? On the **worst days**?
3. How has your condition and its symptoms **changed over time**?
  - a) Do your symptoms come and go? If so, do you know of anything that makes your symptoms better? Worse?
4. How has your condition affected your social interactions, including relationships with family and friends?
5. How has your condition affected your mood (for example; depression, apathy, patience/tolerance for frustration)?

### Topic 2: Patients' perspectives on current approaches to treating Huntington's Disease

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, exercise.)
  - a) What specific symptoms do your treatments 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 treatments improve your ability to do specific activities that are important to you in your daily life?
  - b) How well have these treatments worked for you as your condition has changed over time?
3. 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, interacts with other medications, need to visit your doctor more frequently etc.)
4. Assuming there is no complete cure for your, what would you look for in an **ideal treatment** for a specific aspect of your condition?