Public Meeting on Huntington’s Disease
Patient-Focused Drug Development

September 22, 2015
Welcome

Soujanya Giambone, MBA
Office of Strategic Programs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

September 22, 2015
Agenda

• Setting the context
  – Opening Remarks
  – Overview of FDA’s Patient-Focused Drug Development Initiative
  – Background on Huntington’s Disease and Therapeutic Options
  – Overview of Discussion Format

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

• Discussion Topic 2: Patients’ perspectives on current approaches to treating Huntington’s Disease

• Open Public Comment

• Closing Remarks
Opening Remarks

Billy Dunn, M.D.
Director, Division of Neurology Products (DNP)
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

September 22, 2015
FDA’s Patient-Focused Drug Development Initiative

Theresa Mullin, PhD
Director, Office of Strategic Program
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

September 22, 2015
Patient-Focused Drug Development under PDUFA V

- FDA is developing a more systematic way of gathering patient perspective on their condition and available treatment options
  - Patient perspective helps inform our understanding of the context for the assessment of benefit-risk and decision making for new drugs
  - Input can inform FDA’s oversight both during drug development and during our review of a marketing application

- Patient-Focused Drug Development is part of FDA commitments under the fifth reauthorization of the Prescription Drug User Fee Act (PDUFA V)
  - FDA will convene at least 20 meetings on specific disease areas over the next five years
  - Meetings will help develop a systematic approach to gathering patient input
Identifying Disease Areas for the Patient-Focused Meetings

• In September 2012, FDA announced a preliminary set of diseases as potential meeting candidates
  – Public input on these nominations was collected. FDA carefully considered these public comments and the perspectives of our drug review divisions at FDA

• FDA identified a set of 16 diseases to be the focus of meetings for fiscal years 2013-2015
  – Another public process was initiated and 8 diseases were determined as the disease set for fiscal years 2016-2017
### Disease Areas to be the focus of meetings for FY 2013-2017

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Tailoring Each Patient-Focused Meeting

• Each meeting focuses on a set of questions that aim to elicit patients' perspectives on their disease and on treatment approaches
  – We start with a set of questions that could apply to any disease area; these questions are taken from FDA’s benefit-risk framework and represent important considerations in our decision-making
  – We then further tailor the questions to the disease area of the meeting (e.g., current state of drug development, specific interests of the FDA review division, and the needs of the patient population)

• Focus on relevant current topics in drug development for the disease at each meeting
  – E.g., focus on HIV patient perspectives on potential “cure research”

• We’ve learned that active patient involvement and participation is key to the success of these meetings.
“Voice of the Patient” Reports

• Following each meeting, FDA publishes a Voice of the Patient report that summarizes the patient testimony at the meeting, perspectives shared in written docket comments, as well as any unique views provided by those who joined the meeting webcast.

• These reports serve an important function in communicating to both FDA review staff and the regulated industry what improvements patients would most like to see in their daily life.

• FDA believes that the long run impact of this program will be a better, more informed understanding of how we might find ways to develop new treatments for these diseases.
Background on Huntington’s Disease

Gerald D. Podskalny, DO, MPHS
Division of Neurology Products (DNP)
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
My remarks today do not necessarily reflect the official views of FDA.
Genetic Features of Huntington’s Disease

- Huntington’s disease is an inherited disease
- Prevalence of 2.12/100,000\(^1\) to 10.85/100,100\(^2\)
- Autosomal dominant gene transmission-If a parent has Huntington’s disease, each child has a 50% chance of inheriting the gene
- Genetic (DNA) testing available to detect the presence of an expansion (36 or more CAG repeats) on the short (p) arm of chromosome 4\(^3\).


Clinical Signs and Symptoms

- Onset of symptoms usually between ages 30 and 50 years
  - Juvenile onset (< 20 years)
  - Late onset (onset > 60 years)
- Chorea is the most commonly recognized feature of the disease
- Cognitive Impairment (thinking, memory)
- Behavioral Change (decline in: judgment, mental flexibility, reduced self-care)
- Psychiatric Symptoms (depression, anxiety, apathy, irritability, obsessive/compulsive symptoms)
- Swallowing difficulty
- Weight loss
Treatments

• Current treatments attempt to reduce the symptoms of Huntington’s disease.

• Motor symptoms (chorea)
  – Tetrabenazine (Xenazine)
  – *Neuroleptic drugs (Haldol, Risperidone, and others)

• Antidepressants

• Nutritional support

• Modified diet to make swallowing easier

• Therapy (P.T., O.T.)

• Counseling

* These medications are not approved to treat chorea caused by Huntington’s disease
Family and Caregiver Support

- Genetic counseling
- Psychological counseling
- Support groups
- Respite care
- Family support and communication
Conclusion

- The FDA is aware of unmet medical needs experienced by patients who have Huntington’s disease.

- FDA requested this public meeting to hear comments about the impact of Huntington’s disease on the daily lives of patients, caregivers, and family members.

- Thank you for taking the time and making the trip to share your comments with us today.
Thank You
Overview of Discussion Format

Soujanya Giambone, MBA
Office of Strategic Programs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

September 22, 2015
Discussion Overview

Topic 1: The symptoms that matter most to you

- Which symptoms have the most significant impact on your life?
- How do these symptoms affect your ability to do specific activities?
- How have your symptoms changed?
- How has your condition affected your social interactions?
- How has your condition affected your mood?

Topic 2: Current approaches to treating Huntington’s disease

- What are you doing to treat Huntington’s disease?
- How well is/are the treatment(s) treating your significant symptoms?
- What are the biggest downsides to your treatments?
- What would you look for in an “ideal” treatment?
Discussion Format

• We will first hear from a panel of patients and caregivers
  – The purpose is to set a good foundation for our discussion
  – They reflect a range of experiences with Huntington’s disease

• We will then broaden the dialogue to include patients and patient representatives in the audience
  – The purpose is to build on the experiences shared by the panel
  – We will ask questions and invite you to raise your hand to respond
  – Please state your name before answering
Discussion Format, continued

• You’ll have a chance to answer “polling” questions
  – Their purpose is to aid our discussion
  – In-person participants, use the “clickers” to respond
  – Web participants, answer the questions through the webcast
  – Patients and patient representatives only, please

• Web participants can add comments through the webcast
  – Although they may not all be read or summarized today, your comments will be incorporated into our summary report
  – We’ll occasionally go to the phones to give you another opportunity to contribute
Send us your comments!

- You can send us comments through the “public docket”
  - The docket will be open until November 22, 2015
  - Share your experience, or expand upon something discussed today
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Resources at FDA

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• CDER Office of Center Director
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  – Contact: Christopher Melton, christopher.melton@fda.hhs.gov
  – Facilitates communication and collaboration between CDER and patient and healthcare professional stakeholders and others on issues concerning drug development, drug review and drug safety.
Discussion Ground Rules

- We encourage patients to contribute to the dialogue—caregivers and advocates are welcome too.
- FDA is here to listen.
- Discussion will focus on symptoms and treatments.
  - Open Public Comment Period is available to comment on other topics.
- The views expressed today are personal opinions.
- Respect for one another is paramount.
- Let us know how the meeting went today; evaluation forms at registration desk.
Where do you live?

A. Within Washington, DC metropolitan area (including the Virginia and Maryland suburbs)

B. Outside of the Washington, D.C. metropolitan area
Have you ever been diagnosed as having Huntington’s disease?

A. Yes
B. No
Are you:

A. Male
B. Female
Age:

A. Younger than 20
B. 21 – 30
C. 31 – 40
D. 41 – 50
E. 51 – 60
F. 61 or greater
G. Not applicable
What is the length of time since your diagnosis?

A. Less than 5 years ago
B. 5 years ago to 10 years ago
C. 10 years ago to 20 years ago
D. More than 20 years ago
E. I’m not sure
Do you have a family history of Huntington’s disease?

A. Yes
B. No
C. I’m not sure
Discussion Topic 1

Disease symptoms and daily impacts that matter most to patients

Soujanya Giambone
Facilitator
Topic 1 Panel Participants

• Katie Jackson
• Denise Hudgell
• Marie Clay
• Julie Rosling
• Colleen Walsh-Barnes
Topic 1 Discussion: Disease symptoms and daily impacts that matter most to patients

• Of all the symptoms that you experience because of your condition, which **1-3 symptoms** have the most significant impact on your life?
• 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?
• How has your condition and its symptoms **changed over time**?
• How has your condition affected your social interactions, including relationships with family and friends?
• How has your condition affected your mood?
Of all the symptoms you have experienced because of Huntington’s disease, which do you consider to have the most significant impact on your daily life? Please choose up to three symptoms.

A. Cognitive impairment (such as difficulty concentrating, difficulty with complex tasks)
B. Chorea
C. Fatigue
D. Unsteady gait, difficulty walking
E. Depression or Anxiety
F. Slurred speech
G. Weight Loss
H. Difficulty swallowing
I. Other symptoms not mentioned
Topic 1 Discussion: Disease symptoms and daily impacts that matter most to patients

- Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
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BREAK
Discussion Topic 2

Patients’ perspectives on current approaches to treating Huntington’s disease

Sara Eggers
Facilitator
Topic 2 Panel Participants

- Karen Milek
- Cheryl Sullivan Staveley
- Stacey Sargent
- Karen Douglas
- James D'Ambola
Topic 2 Discussion: Patients’ perspectives on current approaches to treating Huntington’s disease

• What are you currently doing to help treat your condition or its symptoms?
• What specific symptoms do your treatments address?
• How well does your current treatment regimen treat the most significant symptoms of your disease?
• What are the most significant downsides to your current treatments, and how do they affect your daily life?
• Assuming there is no complete cure for your condition, what would you look for in an ideal treatment for your condition or a specific aspect of your condition?
Have you ever used any of the following drug therapies to help reduce your symptoms of Huntington’s disease? **Check all that apply.**

A. Tetrabenazine (Xenazine)
B. Antipsychotic drugs (such as Risperdal, Haldol)
C. Antidepressants
D. Other drug therapies not mentioned
E. I’m not sure
Besides your drug therapies, what other therapies have you used to help reduce your symptoms of Huntington’s disease? Check all that apply.

A. Psychotherapy
B. Speech therapy
C. Physical therapy
D. Occupational therapy
E. Diet modifications
F. Behavioral therapy (such as counseling or support groups)
G. Other therapies not mentioned
H. I’m not using any other therapies
Topic 2 Discussion: Patients’ perspectives on current approaches to treating Huntington’s disease

• What are you currently doing to help treat your condition or its symptoms?
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Additional Resources

• Huntington's Disease Society of America
  – For more information, please call: 1-800-345-4372
Open Public Comment Period
Closing Remarks

Eric Bastings, M.D.
Deputy Director, Division of Neurology Products (DNP)
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
LUNCH
Public Meeting on Parkinson’s Disease
Patient-Focused Drug Development

September 22, 2015
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Soujanya Giambone, MBA
Office of Strategic Programs
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Director, Division of Neurology Products (DNP)
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Susanne R. Goldstein, MD
Division of Neurology Products (DNP)
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
Disclaimer

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Epidemiology of Parkinson’s Disease

- Affects approximately 1 million people in the United States (0.3% of the population)
- Approximately 60,000 new cases diagnosed/year
- Average age of onset 60 years (40-80 years)
- 5-10% of people with PD have symptoms before age 40, young onset Parkinson’s disease (YOPD)
- Prevalence in population >80 years old is 10%
Etiology of Parkinson’s Disease

- Specific causative factor unknown

- Genetic and environmental factors probably involved
  - Genetic factors play a larger role in patients with YOPD
  - Environmental factors play a larger role in patients with onset after age 50
Etiology of Parkinson’s Disease

Potential Causes of Parkinson’s Disease

**Genes**
- α-synuclein
- Parkin
- UCH-L1
- Susceptibility genes

**Environment**
- Pesticides
- Rural living
- Other (?)

**Pathogenic Mechanisms**
- Protein aggregation
- Mitochondrial dysfunction
  - Oxidative stress
  - Inflammation
  - Excitotoxicity

**Apoptosis (cell death)**

UCH-L1 = ubiquitin hydrolase L1.

Clinical Features of Parkinson’s Disease

- **Motor symptoms**
  - Muscular rigidity/stiffness
  - Resting tremors
  - Impaired balance

- **Non-motor symptoms**
  - Mood disorders (depression, anxiety)
  - Memory difficulty
  - Hallucinations
  - Difficulty sleeping
Treatments (Medical/Surgical)

- **Pharmacologic (Drugs)**
  - Carbidopa-levodopa (Sinemet, Sinemet CR, Rytary)*
  - Carbidopa-levodopa infusion (Duopa)*
  - Dopamine agonists (Mirapex, Requip, Neupro, Apokyn)*
  - COMT inhibitors (Comtan, Stalevo, Tasmar)*
  - MAO-B inhibitors (Eldepryl, Zelapar, Azilect)*
  - Anticholinergics (Artane, Cogentin)*
  - Amantadine (Symmetrel)*
  - Antidepressants/anxiolytics
  - Neuroleptic drugs
    (*Approved for the treatment of PD)

- **Surgical**
  - Deep brain stimulation surgery
Non-pharmacologic Treatments

- Physical/Occupational Therapy
- Speech and Swallowing
- Diet/Exercise
- Counseling
- Caregiver
- Support groups
Conclusion

• The FDA is aware of unmet medical needs experienced by patients who have Parkinson’s disease.

• FDA is conducting this public meeting to hear comments about the impact of Parkinson’s disease on patients, caregivers and family members.

• Thank you for taking the time, and making the trip to share your comments with us today.
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Soujanya Giambone, MBA
Office of Strategic Programs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
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A. Within Washington, D.C. metropolitan area (including the Virginia and Maryland suburbs)
B. Outside of the Washington D.C. metropolitan area
Have you ever been diagnosed as having Parkinson’s disease?

A. Yes
B. No
Are you:

A. Male
B. Female
Age:

A. Younger than 30
B. 30 – 40
C. 41 – 50
D. 51 – 60
E. 61 – 70
F. 71 or greater
What is the length of time since your diagnosis?

A. Less than 5 years ago
B. 5 years ago to 10 years ago
C. 10 years ago to 20 years ago
D. More than 20 years ago
E. I’m not sure
Discussion Topic 1

Disease symptoms and daily impacts that matter most to patients

Soujanya Giambone
Facilitator
Topic 1 Panel Participants

• Daniel Lewis
• Rebecca Houde
• Todd Hebb
• Karl Robb
• Rosa Kim
Topic 1 Discussion: Disease symptoms and daily impacts that matter most to patients

• Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
• 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?
• How has your ability to cope with symptoms changed over time?
• What worries you most about your condition?
• How has your condition affected your social interactions, including relationships with family and friends?
Of all the symptoms you have experienced because of Parkinson’s disease, which do you consider to have the most significant impact on your daily life? Please choose up to three symptoms.

A. Motor symptoms (such as slowed movement, tremor)
B. Impaired balance and coordination
C. Constipation
D. Sleep issues (such as falling asleep, staying asleep or abnormal dreams)
E. Cognitive impairment (such as difficulty concentrating, difficulty with complex tasks)
F. Fatigue and loss of energy
G. Difficulty swallowing/drooling
H. Depression or anxiety
I. Other symptoms not mentioned
Topic 1 Discussion: Disease symptoms and daily impacts that matter most to patients

- Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
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- What worries you most about your condition?
- How has your condition affected your social interactions, including relationships with family and friends?
Discussion Topic 2

Patients’ perspectives on current approaches to treating Parkinson’s disease

Sara Eggers
Facilitator
Topic 2 Panel Participants

- Bill Patterson
- Steven DeWitte
- Gary Rafaloff
- Jenifer Raub
- Kevin Kwok
Topic 2 Discussion: Patients’ perspectives on current approaches to treating Parkinson’s disease

• What are you currently doing to help treat your condition or its symptoms?
• How well does your current treatment regimen treat the most significant symptoms of your disease?
• What are the most significant downsides to your current treatments, and how do they affect your daily life?
• Assuming there is no complete cure for your condition, what would you look for in an ideal treatment for your condition or a specific aspect of your condition?
Have you ever used any of the following drug therapies to help reduce your symptoms of Parkinson’s disease? Check all that apply.

A. Carbidopa-levodopa (such as Duopa, Sinemet, Rytari Stalevo)
B. Dopamine agonists (such as Parlodel, Mirapex, Requip)
C. Monoamine oxidase B (MAO-B) inhibitors (such as Eldepryl, Azilect, Zelapar)
D. Catechol-O-methyltransferase (COMT) inhibitors (such as Comtan, Stalevo, Tasmar)
E. Anticholinergics (such as Artane, Cogentin)
F. Amantadine (Symmetrel)
G. Deep brain stimulation (DBS)
H. Drug therapy to treat hallucinations, depression and/or anxiety
I. Other drug therapies not mentioned
Besides your drug therapies, what other therapies have you used to help reduce your symptoms of Parkinson’s disease? Check all that apply.

A. Exercise
B. Diet and nutrition modifications
C. Counseling and support groups
D. Physical therapy
E. Alternative therapies (such as massage, yoga, acupuncture)
F. Other therapies not mentioned
G. I’m not using any other therapies
Topic 2 Discussion: Patients’ perspectives on current approaches to treating Parkinson’s disease

- What are you currently doing to help treat your condition or its symptoms?
- How well does your current treatment regimen treat the most significant symptoms of your disease?
- What are the most significant downsides to your current treatments, and how do they affect your daily life?
- Assuming there is no complete cure for your condition, what would you look for in an ideal treatment for your condition or a specific aspect of your condition?
Additional Resources

• National Parkinson’s Foundation
  - For more information, please call NPF helpline: 1.800.4PD.INFO (473-4636)
Open Public Comment Period
Closing Remarks

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