

A photograph of several orange, round pills scattered on a white surface. One pill is in sharp focus in the lower right foreground, while the others are blurred in the background.

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**

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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

Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Year 2016-2017
<ul style="list-style-type: none"> • Chronic fatigue syndrome/myalgic encephalomyelitis • HIV • Lung cancer • Narcolepsy 	<ul style="list-style-type: none"> • Sickle cell disease • Fibromyalgia • Pulmonary arterial hypertension • Inborn errors of metabolism • Hemophilia A, B, and other heritable bleeding disorders • Idiopathic pulmonary fibrosis 	<ul style="list-style-type: none"> • Female sexual dysfunction • Breast cancer • Chagas disease • Functional gastrointestinal disorders • Huntington’s disease and Parkinson’s disease (September 22) • Alpha-1 antitrypsin deficiency (September 29) 	<ul style="list-style-type: none"> • Non-tuberculous mycobacterial lung infections (October 15) <p><i>To be announced</i></p> <ul style="list-style-type: none"> • Alopecia areata • Autism • Hereditary angioedema • Patients who have received an organ transplant • Psoriasis • Neuropathic pain associated with peripheral neuropathy • Sarcopenia

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.**

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- 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.

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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

September 22, 2015

*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 2.12/100,000¹ to 10.85/100,100²
- 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 4³.

¹Sipilä JO, Hietala M, Siitonen A, Päivärinta M, Majamaa K. Epidemiology of Huntington's disease in Finland. *Parkinsonism Relat Disord.* 2015 Jan;21(1):46-9. doi: 10.1016/j.parkreldis.2014.10.025. Epub 2014 Nov 3. PubMed PMID: 25466405.

²Squitieri F, Griguoli A, Capelli G, Porcellini A, D'Alessio B. Epidemiology of Huntington disease: first post-HTT gene analysis of prevalence in Italy. *Clin Genet.* 2015 Feb 18. doi: 10.1111/cge.12574. [Epub ahead of print] PubMed PMID: 25689972.

³Huntington's Disease Collaborative Research Group. A novel gene containing a trinucleotide repeat that is expanded and unstable on Huntington's disease chromosomes. The Huntington's Disease Collaborative Research Group. *Cell* 1993;72:971-983.

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 anxiety (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

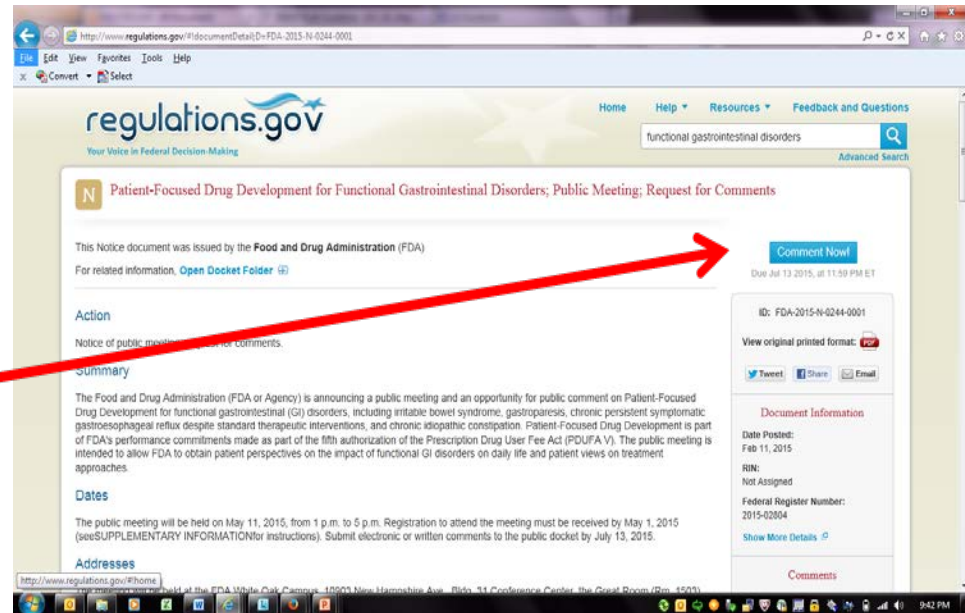
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- **You can send us comments through the “public docket”**
 - The docket will be open until November 22, 2015
 - Share your experience, or expand upon something discussed today
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Visit:

<http://www.regulations.gov/#!documentDetail;D=FDA-2012-N-0967-0747>

Click Comment Now!



Resources at FDA

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 - Contact: PatientNetwork@fda.hhs.gov, (301) 796-8460
 - Liaison between FDA and stakeholder organizations
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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, 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 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



BREAK



Discussion Topic 2

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

Soujanya Giambone
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



Open Public Comment Period

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Closing Remarks

Eric Bastings, M.D.

Deputy Director, Division of Neurology Products (DNP)
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Billy Dunn, M.D.

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Theresa Mullin, PhD

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Background on Parkinson's Disease

Susanne R. Goldstein, MD

Division of Neurology Products (DNP)
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

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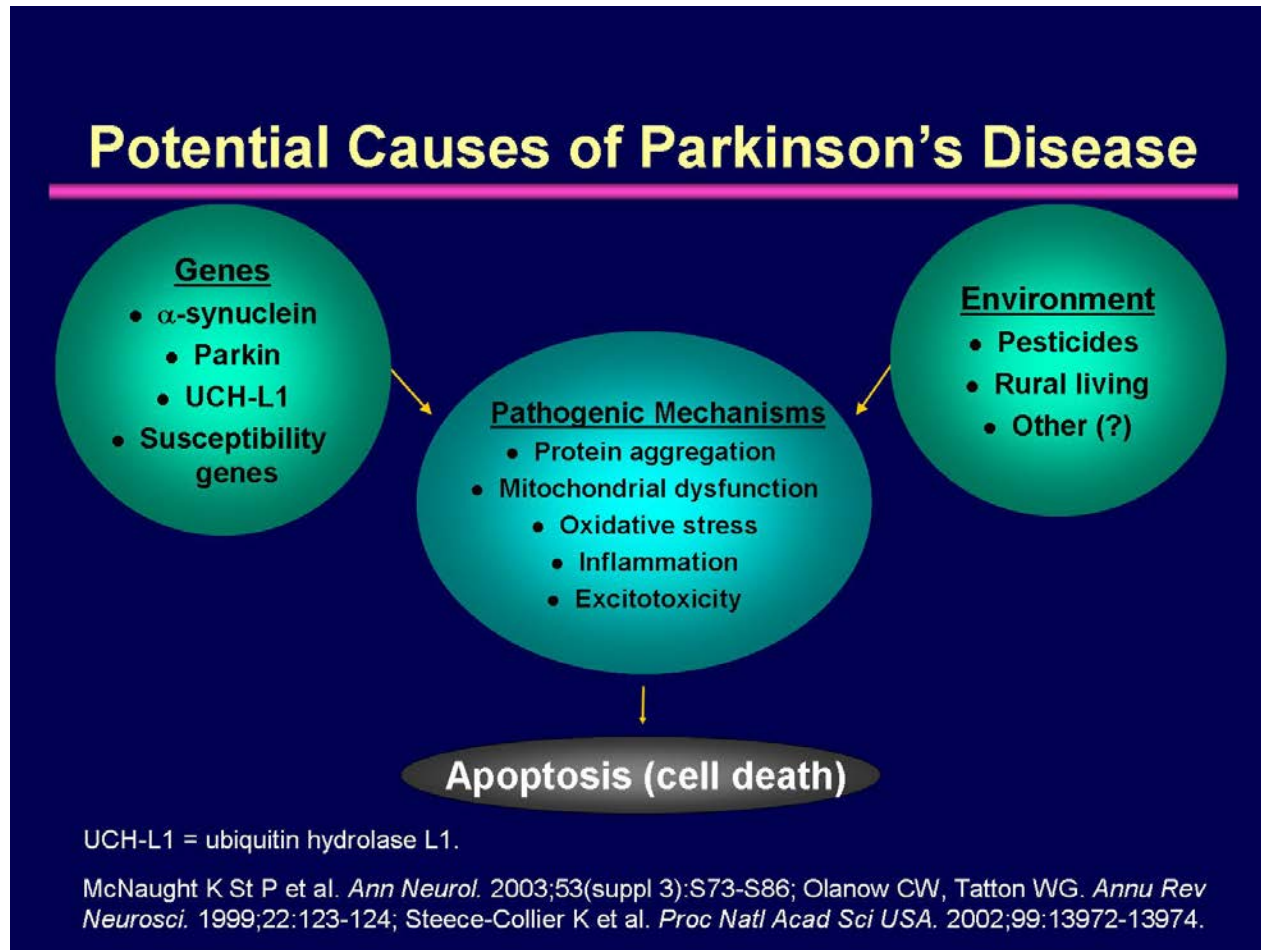
Epidemiology of Parkinson's Disease

- 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



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.

Thank You

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Click Comment Now!

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Resources at FDA

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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?

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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

BREAK

Discussion Topic 2

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

Soujanya Giambone

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

Open Public Comment Period

Closing Remarks

Eric Bastings, M.D.

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