The FDA is listening: Integrating the Voice of the Patient in Drug Development for Parkinson's and Huntington's Diseases

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Objective and Background

Objective

To present highlights from the Patient-Focused Drug Development (PFDD) meetings held at FDA for Parkinson's disease (PD) and Huntington's disease (HD) to raise awareness of the importance of listening to the needs of those living with movement disorders

Background

- Historically, drug development has not systematically incorporated patients' perspectives and preferences into the process
- FDA's Patient-Focused Drug Development initiative, a commitment under the fifth authorization of the Prescription Drug User Fee Act (PDUFA V), aims to more systematically gather patients' perspectives on their condition and available therapies
- As part of this commitment, FDA hosted a public PFDD meeting focused on HD and PD on September 22, 2015, to gain patient feedback on two key topics:
  1. Disease symptoms and daily impacts that matter most to patients
  2. Patient perspectives on treatments
- Participants included patients, caregivers, and advocates

Methods

- The FDA heard directly from participants with an emphasis on symptoms, existing treatment options, and the unmet need for new therapies
- Parkinson's disease
  - 100 in-person participants
  - >160 joined remotely
  - 2/3 of the participants who attended in person had a diagnosis of PD
- Huntington's disease
  - 50 in-person participants
  - 60 participants provided input through the live webcast and polling questions
- A total of 139 Public Docket comments were submitted to the FDA that included comments from caregivers, patients, patient representatives, and organizations representing both the PD and HD communities:
  - Parkinson's Action Network, 23andMe, Parkinson's Disease Foundation, Patients Like Me, and Huntington's Disease Society of America

Results

Participants in the Parkinson's disease meeting emphasized:

- PD is a progressive disease with unexpected onset and which negatively impacts all aspects of patients' lives.
- **Topic 1:** *Disease symptoms and daily impacts that matters most* (**Figure 1**)
  - Motor symptoms, sleep disturbances, cognitive impairment, fatigue, and constipation
  - Increasing reliance on others, inability to perform work activities leading to isolation and negative impact on relationships
- **Topic 2:** *Patient perspectives on treatments for PD*
  - Existing medications have benefits that become limited over time because of adverse effects caused by medications and advancing PD. There is urgent need for medications that are effective in delaying the onset or slowing the progression of symptoms.
  - Ideal treatment would be a once a day pill that controls all symptoms without side effects and a decrease in motor fluctuations such as ON/OFF times
  - Progress of research is priority with hopes for disease prevention

Participants in the Huntington's disease meeting emphasized:

- HD impacts all aspects of patients' lives.
- **Topic 1:** *Disease symptoms that matter most to patients* (**Figure 1**)
  - Cognitive impairment, speech impairments, dystonia, depression and anxiety, sleep issues, gastrointestinal issues, neuropathy, weight loss
  - HD burden leads to inability to perform many, if not all activities leading to loss of independence and increased reliance on others for care
  - HD has a devastating impact on relationships, and there is worry of passing the disease on to their children
- **Topic 2:** *Patient perspectives on treatments for HD*
  - People with HD report using many different drugs to treat symptoms. Current treatments do not prevent worsening of their most disabling symptoms.
  - The benefits of non-drug therapies, such as exercise, dietary modifications, lifestyle changes (to minimize stress), meditation, and prayer
  - There is urgent need for medications that are effective in delaying the onset of symptoms or slowing the progression of symptoms
  - Improved research on a cure for HD (including gene silencing therapies and stem cell therapies), faster clinical trials and drug development, and expedited drug reviews

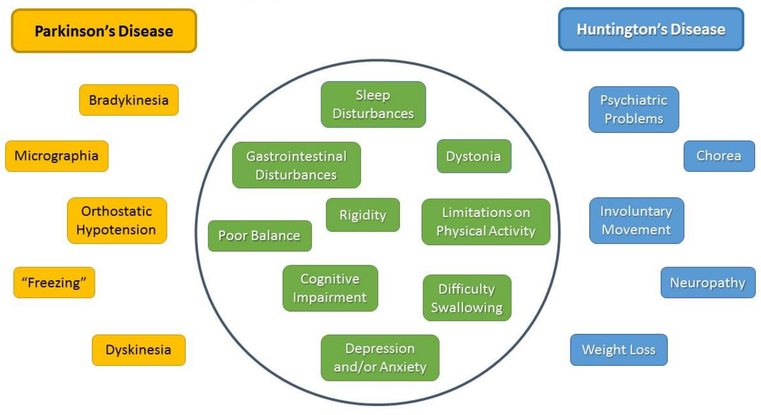

Figure 1 **Significant symptoms identified by PFDD participants**

"Voice of the Patient" Report

- Following each meeting, the FDA published a **Voice of the Patient** report that summarizes the patient testimonies at the meeting. Perspectives shared in written docket comments, as well as any unique views provided by those who joined the meeting webcast. The full webcast is available on the FDA website (**Ref. 1**; **LINK** above) for viewing.
- These reports serve an important function in communicating to both the FDA and regulated industry what improvements patients would most like to see in their daily lives
- FDA believes that the long-term impact of this program will be a better, more informed understanding of the patients' needs

FDA Views

- FDA recognizes that patients have a unique ability to contribute to our understanding of the broader context of these diseases. This information is important to the assessment of new therapies being considered for approval.
- The insight provided during these meetings will aid in FDA's understanding of what patients truly value in a treatment and inform the agency's evaluation of the benefits and risk of future treatments for PD and HD patients (**Figure 2**).

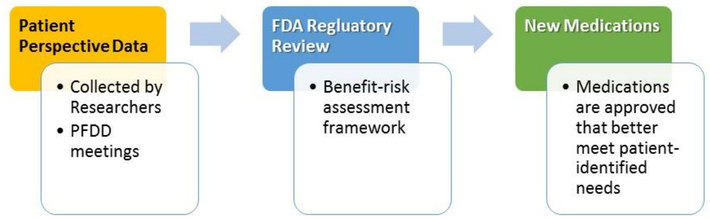

Figure 2 **Use of patient data in FDA regulatory decision-making for new medications**

Critical Path Institute (C-Path)

- Critical Path Institute is a nonprofit organization supported by FDA with the mission of catalyzing the development of new therapies to advance medical innovation and regulatory science. This is achieved by leading teams that share data, knowledge and expertise resulting in sound, consensus based science. At present, C-Path hosts fourteen distinct consortia, the following two focused on movement disorders.

Parkinson's Disease

- The *Critical Path for Parkinson's (CPP) Consortium*, founded in 2015 by Parkinson's UK and C-Path, is a worldwide initiative founded on precompetitive data sharing as the foundation to tackle a broad array of issues relevant to PD drug development (**Ref. 2**)

CPP PARKINSON'S CONSORTIUM CRITICAL PATH INSTITUTE

Huntington's Disease

- C-Path and the CHDI Foundation are presently collaborating to set up a broad-based *Huntington's Disease Regulatory Science Consortium* (HD-RSC) to more clearly define regulatory pathways to HD therapies
- Bringing together industry, regulatory authorities, academics, and patient advocates to share data and expertise with the aim of advancing regulatory endorsed drug development tools and clinical data standardization

HD-RSC HUNTINGTON'S CONSORTIUM CRITICAL PATH INSTITUTE

Conclusions and Next Steps

- The PFDD meeting allowed the FDA to learn what matters most to patients and caregivers regarding symptoms, impacts and aspects of treatments. This input supports benefit-risk assessments for products under review and aides worldwide consortia to focus on the needs of the community.

References

(1) FDA Patient Focused Drug Development Website and meeting materials: http://www.fda.gov/Drugs/NewsEvents/ucm451807.htm (also see **LINK** above)

(2) Stephenson D., Hu M.T., Romero K., Breen K., Burn D., Ben-Shlomo Y., Bhattaram A., Isaac M., Venuto C., Kubota K., Little M.A., Friend S., Lovestone S., Morris H.R., Grosset D., Sutherland M., Gallacher J., Williams-Gray C., Bain L.J., Avilés E., Marek K., Toga A.W., Stark Y., Forrest Gordon M., Ford S., Precompetitive data sharing as a catalyst to address unmet needs in Parkinson's disease. J. Parkinson's Dis. 5:581-594, 2015

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