

The FDA is Listening: Integrating the Voice of the Patient in Drug Development for Parkinson's Disease

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Background and Objectives

Background:

- Parkinson's disease (PD) has a debilitating effect on patients lives and there is increasing recognition on the role of both motor and non-motor symptoms in terms of burden and quality of life.
- Traditional drug development has not systematically incorporated patients' perspectives and preferences into the process.
- FDA's Patient-Focused Drug Development (PFDD) 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.
- Critical Path for Parkinson's (CPP) consortium (Figure 1) is a public-private-partnership sponsored by Critical Path Institute, Parkinson's UK, and industry aimed at facilitating the path of PD drug development.



Figure 1 CPP Members – Enabling Advancement of PD Drug Development

- On September 22, 2015, the FDA sponsored the PFDD meeting focused on Parkinson's disease. Discussion focused on two key topics:

- The effects of PD that matter most to patients
- Patients' perspectives on treatments for PD

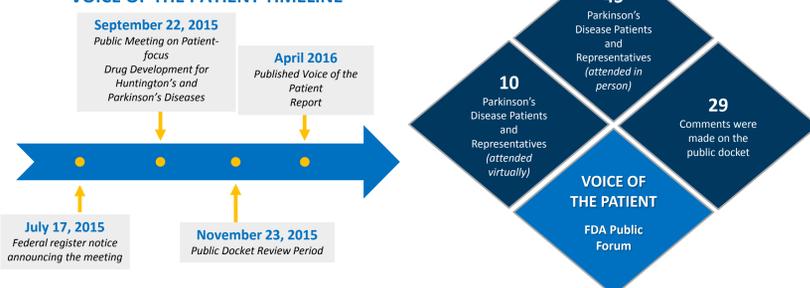
Objectives:

- To present the highlights from the PFDD meeting held at the FDA for Parkinson's disease to raise awareness of the needs of those living with PD.

Methods

- FDA's Patient Focused Drug Development aims to more systematically gather patients' perspectives on their condition and available therapies to treat their condition.
- The Parkinson's disease focused PFDD meeting was organized with the following attributes:

VOICE OF THE PATIENT TIMELINE



Results

Meeting participation: One hundred participants attended the FDA's PFDD meeting in person and >160 joined the four hour meeting remotely. Two-thirds of the participants who attended in person were diagnosed with PD.

Meeting format:

- A panel of patients and patient representatives shared comments to begin the dialogue.
- Panel comments were followed by facilitated discussion inviting comments from other participants.
- An FDA facilitator led the discussion and a panel of FDA staff (10) asked follow-up questions.
- Participants who joined the meeting via webcast were able to contribute comments.
- In person and web participants were periodically invited to respond to polling questions.
- Gathering of public input was extended for two months past the date of the meeting and all feedback was compiled to prepare a voice-of-the-patient report that has been made publically available.



- Participants in the PD PFDD meeting represented a range of experiences with its symptoms and treatments.
- The FDA heard directly from participants with an emphasis on symptoms and the limitations of existing treatment options.
- Participant testimonials focused on quality of life issues and the impact of PD on daily life.

TOPIC 1: Disease Symptoms and Daily Impacts that Matter most to Patients

- Perspectives on most significant symptoms
 - Motor symptoms
 - Cognitive impairment
 - Sleep disturbances
 - Other symptoms
- Overall impact of Parkinson's disease on daily life
 - Reliance on others
 - Ability to perform at work
 - Isolation and impact on relationships

TOPIC 2: Patient perspectives on treatments for PD

- Prescriptions and over the counter drugs
- Limited or decreased benefit over time and harmful side effects
 - Non drug therapies

Exercise regimens include training, rowing, walking, cycling, hiking, dancing, Tai Chi, yoga

Off time, pill burden, balance with food intake, limited access to treatment options

- Perspectives on an ideal treatment for Parkinson's disease
- The perfect treatment would be a once-a-day pill that controls all symptoms, minus side effects without on-off times**

Comments submitted to the Public Docket:

[Patients, Caregivers, Parkinson's Disease Foundation, Twenty three and Me, Patients Like Me]

Input on symptoms, treatment and unmet needs similar to input at the public meeting, emphasizing the burdensome nature of PD.

Specific comments highlighted below:

- Triggers that worsen symptoms: Stress, cold weather, fatigue, lack of sleep and changes in physical health.
- Impact of PD on daily life: Lack of ability to perform activities, "almost all extracurricular activities such as sports and training have come to complete stop".
- Negative perception of PD patients by family members or public: Commenter stated they were "treated as a child" due to PD symptoms.

"Voice of the Patient" Report:

- 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 the patients' expectations for to develop new treatments for these diseases.

Conclusions

- By ensuring a thorough understanding of the impact of the diverse manifestations of PD from a patient perspective, the FDA's PFDD initiative is intended to identify unmet needs from the patient perspective, and to include their perspective in the FDA's consideration of new treatments for PD.
- The insight provided during the meeting will aid in FDA's decision-making as it establishes the context in which it considers the risks and benefits associated with future treatments for PD patients.
- This goal aligns with those of the Critical Path for Parkinson's (CPP) consortium, to accelerate development of effective treatments for those with PD through a robust multinational collaboration centered on data sharing.

References

- FDA Patient Focused Drug Development Website and meeting materials: <http://www.fda.gov/Drugs/NewsEvents/ucm451807.htm>
- 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