Partnering with People with Lived Experience in the Critical Path for Parkinson's Consortium

There is hope for better treatments for Parkinson's Disease (PD) that could slow or stop the progression of the disease. However, when testing novel drugs in clinical trials we urgently need for more sensitive, patient-centered trial endpoints – measures of how effective drugs are – to advance the clinical development through FDA approval.

As a person affected by PD, **your participation is critical** to inform research projects developing better measures - endpoints - that will determine whether new treatments have an impact on **aspects of Parkinson's that are meaningful to people living with the disease. And these endpoints could change the way that everyone studying Parkinson's evaluates aspects of the condition.**

Who we are: Global collaboration to pave the way to new treatments

Critical Path Institute (C-Path) is a nonprofit organization supported by the FDA, which exists to make it faster, less expensive, and more efficient to get new therapies to people living with the disease.

The Critical Path for Parkinson's (CPP) Consortium, founded in 2015 by the Parkinson's research and support organization, Parkinson's UK, alongside C-Path, is a global initiative aiming to **tackle issues relevant to PD drug development**. CPP also collaborates with academic researchers, clinicians, drug developers, and global Parkinson's advocacy organizations.

What we want to achieve: Advancement of Patient-Centric Endpoints

The current endpoint that is used to assess the effects of PD therapies and is approved by regulators such as the FDA, MDS-UPDRS, has been effective for many trials of symptomatic treatments, yet there is a need for new instruments to detect individual progression patters because, as we know, Parkinson's is such an individual disease. CPP currently facilitates a dedicated Working Group that aims to **align with regulatory agencies** like FDA on meaningful patient-centric endpoints for use in PD clinical trials. The objective is to share resources and learnings to find a common solution in line with FDA's Patient-Focused Drug Development guidance. The Working Group consists of experts from pharmaceutical companies, academia, research foundations, Parkinson's advocacy organizations, people living with PD, as well as FDA.

How you can help us: The role you will play

Share your experiences living with PD and learnings about existing measures and recommendations for future outcome measures that matter to you. Your experience with Parkinson's day in and out makes you an expert – a representative with an equal voice to research scientists invested in PD.

Knowing if you are a good fit: Who makes an ideal representative?

People affected by PD across the continuum of the disease, including people with Parkinson's and care partners, will provide important perspectives. People who can also share perspectives from a broader network of people affected by Parkinson's can further their impact.

We are seeking a diverse group of individuals who are united in the mission to share voices and ideas to affect change in the development of meaningful therapeutics. This group will form the CPP-PPP (Partnering with People with Parkinson's) panel. The following are ideal characteristics for a scientist, but are not required to become involved:

- Living with Parkinson's at any stage of the disease, with or without motor symptoms
- Individuals living in the US, UK, or Canada
- Direct experience in PD-related research and/or clinical trials in the scientific, public health, or academic field
- Comfortable with sharing their PD journey and symptoms with researchers and other stakeholders
- Experience with advocacy initiatives or collaborating with PD groups
- Knowledge of the current clinical trial landscape for PD, drug development, or related research

For care partners, we hope to collaborate with those who have the following experience, although not required to participate:

- Experience living with a person with PD as a care partner
- Experience participating in PD-related research
- Experience with Parkinson's advocacy initiatives
- Experience with Parkinson's related collaboration

Structure and scope of participation: Activities you will be engaged in

PwP contributors and care partners recruited for membership on the Partnering with People with Parkinson's panel will serve a term of 3-6 months and will receive a stipend for participation. The duration of the council will be for a total of two years. During this time, we would like to engage with you by:

- Participating in 1-hour virtual call every 2-3 months with other council members and non-profit organizations
- Developing a collective narrative to publish in Journal of Parkinson's Disease annual summary
- Developing messaging for the broader PD community (e.g., writing one blog piece annually to share on the CPP website: (https:/c-path.org/programs/cpp/)
- Convening a meeting prior to the World Parkinson's Congress (WPC) in July
- Convening a meeting during the WPC to identify themes for discussion.
- Contributing to posters and papers; helping to develop further outreach and information dissemination plans.
- Engaging in discussions around the following topics:
    - Data standards, sharing principles, and modeling.
    - Defining successful endpoints (lessons learned in case examples)
    - And more

Collectively, these activities will help us achieve the overarching goal of addressing the question: **How do we decide on a meaningful measure?** CPP will share briefing materials prior to each meeting and will

distribute notes within two weeks post-meeting. Council members will be invaluable in providing input on agenda topics and feedback on meeting notes.

How to get involved?

To be considered for this role, please fill out the interest form located on our partner organizations' websites.

Our CPP Partner Organizations are key liaisons:

- The Michael J. Fox Foundation
- Parkinson Canada
- Parkinson's UK
- Cure Parkinson's
- The Parkinson's Foundation
- Parkinson's Europe

We look forward welcoming you to this important initiative!

Useful References (Patient-Focused Drug Development)

Dr. Sonia Mathur's blog, "Partnering in Research" Feb 1, 2022
https://www.unshakeablemd.com/post/partnering-in-research

*EMA/FDA focus on Patient-Focused Drug Development
Mavris M, Furia Helms A, Bere N. Engaging patients in medicines regulation: a tale of two agencies. Nat Rev Drug Discov. 2019 Nov;18(12):885-886. doi: 10.1038/d41573-019-00164-y.

Michael J Fox Foundation hosted webinar, June 2021
Novel Instruments to Capture Patient Outcomes in Parkinson's
https://share.vidyard.com/watch/f135GsHZfrTAsPwLn6UFML

*Murphy A, Bere N, Vamvakas S and Mavris M (2022) The Added Value of Patient Engagement in Early Dialogue at EMA: Scientific Advice as a Case Study. Front. Med. 8:811855. doi: 10.3389/fmed.2021.811855

*Nature Medicine review, Jan 2022
The value of patient-reported outcomes in early-phase clinical trials
https://www.nature.com/articles/s41591-021-01648-4?utm_source=xmol&utm_medium=affiliate&utm_content=meta&utm_campaign=DDCN_1_GL01_metadata