

Bringing Patient Engagement into Research and Discovery Phase

UCB Collaborates with the Parkinson's Foundation and Parkinson's UK to Apply CTTI's Patient Group Engagement Recommendations

SUMMARY

UCB, the Parkinson's Foundation, and Parkinson's UK collaboratively used CTTI's Patient Group Engagement recommendations and prioritization tool to prioritize focus topics for a global patient engagement council that embeds participant insights into the very beginning of research.

GOAL(S)

Parkinson's disease (PD) is the second most common neurodegenerative disease, with nearly one million individuals living with PD and approximately 60,000 Americans diagnosed each year. The patient experience with PD is unique, and yet in early 2021, no best practice framework existed to embed patient voices into the PD research and development process at an early stage – despite mounting evidence that such a framework can better address community needs and priorities, utilize stronger patient-reported outcomes and meaningful endpoints, reduce study burden, and enhance recruitment and retention.

Three PD-focused organizations decided to change that. UCB, a global pharmaceutical company, had an existing relationship with the Parkinson's Foundation and Parkinson's UK, but the three groups wanted to deepen their engagement. They agreed to form a global Patient Engagement Council for Parkinson's Research together with five strategic thinkers living with Parkinson's (Patient Experts) who could represent the wider community with an initial remit to ensure that patient insights are central to the overall strategy and activities across UCB's Parkinson's disease research and early clinical development program. The Council's long-term vision is that all therapeutics for Parkinson's be developed by a fully integrated partnership between all key relevant stakeholders.

CHALLENGES

UCB, the Parkinson's Foundation, and Parkinson's UK needed a charter and governance model to help them formalize such a collaboration, which is not common in the early research and development stage. In addition, while the primary interest of all three organizations and the Patient Experts aligned – to bring new therapies to people living with Parkinson's – they needed a way to identify and prioritize topics that would have the biggest impact for them.

SOLUTION(S)

CTTI's suite of Patient Group Engagement (PGE) [recommendations and resources](#) support the clinical research community in understanding the value of patient engagement. Its [prioritization tool](#) for Sponsors and Patient Groups was built to help patient groups and clinical research sponsors identify high-value opportunities to work together. UCB, the Parkinson's Foundation, Parkinson's UK, and the Patient Experts used it as a foundational tool to help support decisions on what topics should be progressed first.

TAKING ACTION

The inaugural meeting of the global Patient Council took place in late September 2021, where the group set out its broad goals and objectives as follows:

1. Co-create a patient-centered standard model of research and development that meets the needs and priorities of people living with Parkinson's;
2. Develop insights that can be shared across the Parkinson's community to improve Parkinson's research and avoid duplication of efforts; and
3. Elevate the role of patient engagement within the international Parkinson's research community, enabling increased opportunities for collaboration.

The Chief Executive Officers of the Parkinson's Foundation, Parkinson's UK, and UCB attended the inaugural meeting together with additional representatives from all parties, including experts from research, patient-reported outcomes, early discovery, medical, and patient voices.

With the foundational goals in place, the Council walked through CTTI's PGE work and the Patient Focused Medicines Development (PFMD) [Patient Engagement Management \(PEM\) Suite](#) and discussed how these tools could be used to embed patient community engagement across the clinical trial continuum.

Each Council member set about submitting their top two priority topics which were then bucketed under three key headings: Research, Clinical Development, and Real-Life Setting. Everyone independently ranked the topics using CTTI's prioritization tool. The Patient Experts decided to meet separately to discuss their findings and to reach a consensus, which they then shared with the Council for greater discussion.

The Council members came together and explored each topic with granularity to clarify the group's understanding of each. For example, why is a "disease-modifying therapy" narrative important? What does a Target Patient Value Profile* (TPVP) actually deliver? When we talk about "investment," are we referring to time investment or is it simply financial investment? Attendees participated in a debate to ensure a holistic perspective from both the patient groups, Patient Experts, and UCB before locking in any priorities. Given that nearly all the topics on the table were important, this prioritization was tricky – there had to be agreement that a higher priority ranking for one topic did not devalue another.

The results from this exercise were:

Areas considered high/high-moderate benefit across all members:

- Patient-informed TPVP*
- Shared understanding of disease modifying therapies
- Patient-informed risk/benefit
- Outcomes and endpoints
- Granularity of real-world evidence
- Optimizing clinical trial design

Areas considered low prioritization OR too high investment (for now) by all:

- Evaluation of non-motor scales
- Decentralized research and traditional clinical trials
- Health diversity, equity, and inclusion (the Council agreed that this will be embedded as a consideration throughout all work, but to consider as a standalone project is not achievable at the moment)

Areas of contrasting opinions across members:

- Patient stratification
- Increasing the patient voice in regulatory

Then, they refined further by considering the priorities through three new lenses: 1) what topics are low-hanging fruit (those that can make a meaningful difference to people with PD that can be achieved with low investment and a short timeline; 2) what topics have a specific focus on research; and 3) what topics can provide immediate support to UCB's Research and Discovery (R&D) programs.

At the end of this assessment, the Council reached consensus on three priority topics for focus in late 2022 and into 2023:

1. Development of a patient co-creation process for all TPVP*.
2. Development of a narrative to enhance understanding among the patient community on what is meant by "disease-modifying therapies" and how R&D is moving at a rapid pace with potentially new opportunities.

Enhancing diversity, equity, and inclusion across an early UCB asset.

IMPACT

The global [Patient Engagement Council for Parkinson's Research](#) has helped all parties work together more strategically with a bird's eye view of priorities. This more holistic perspective has helped the Council look at its research through a long-term lens to ensure the projects and priorities UCB invests in are right-tracked to bring the most benefit to people living with PD. To further embed the patient voice in decision-making, the Council is currently exploring how they can integrate patients with lived experience more firmly into their regulatory and Health Technology Appraisal discussions. In addition, the Council has welcomed five people who live with PD to guide its work – each of them has a very personal Parkinson's story and reason for wanting to be involved with this group:

One of those is a patient advocate from the Netherlands, diagnosed with PD in 2018:

"When I first learned I had Parkinson's my world collapsed, and along the way I managed to accept this fact. But acceptance is not resignation. I will fight this disease until the end. By joining the Patient Engagement Council for Parkinson's Research, I can extend this fight and make it a collective battle of many against this terrible disease. Finding new therapies or even a cure against Parkinson's is all about co-creation of the Parkinson's community. Patients, healthcare professionals, pharmaceutical companies, and foundations have to come together to make a stand. The Council is a beautiful example of the power of co-creation to join forces!"

ADVICE

UCB, the Parkinson's Foundation, and Parkinson's UK suggest using the CTTI Prioritization Tool as a starting point, but stress that the process could benefit from a deeper assessment to clarify how priorities are defined and to better understand other stakeholders' perspectives. Ideally, groups should ensure clarity and full understanding of the topics prior to using the tool, then engage in dialogue afterward to refine the priorities. Also, be careful to ensure equal weight from all parties involved. For example, when the Patient Council combined priorities from all meeting attendees, it realized the responses were weighted toward UCB, who had more representatives at the table. Ensure fair representation and invest in the dialogue to improve the research. While the CTTI Prioritization Tool is a foundational starting point, the power to yield better, more patient-centric trials lies in the tool's ability to unlock dialogue and different perspectives across stakeholders.

** The purpose of a Target Patient Value Profile (TPVP) is to ensure that the drug development process is efficient and provides all the required relevant medical, technical, and scientific information for evaluating the viable outcome of a potential new medicine. Historically, the early design of TPVP's was largely driven by joint insights from clinical scientists, desk research, and conversations with expert healthcare professionals, with little or no participation of people living with*

the condition.

ORGANIZATION

UCB Pharma
Parkinson's Foundation
Parkinson's UK

CONTACT

Kate Trenam 

ORGANIZATION TYPE

Industry
Patient

IMPLEMENTATION DATE

2021

TOPIC

Patient Engagement

RELATED CTTI PROJECT

[Patient Group Engagement](#)

CTTI RESOURCES

[CTTI Recommendations: Effective engagement with patient groups around clinical trials](#)

ADDITIONAL RESOURCES

[UCB's Patient Engagement Council for Parkinson's Research \(website\)](#)

[World Parkinson's Day 2022 - A patient centered model for Parkinson's research](#)

[Introduction to UCB's Patient Engagement Council for Parkinson's Research](#)