CRITICAL PATH INSTITUTE

Huntington's Disease Awareness Month: Advancing Drug Development Through Lived Experience

Each May, Huntington's Disease Awareness Month serves as an important opportunity to raise visibility for Huntington's disease (HD) and recognize the urgent need for continued scientific innovation, advocacy, and collaboration in the pursuit of meaningful therapies for individuals and families affected by this devastating condition.

Huntington's disease is a rare, inherited neurodegenerative disorder characterized by progressive motor, cognitive, and neuropsychiatric symptoms. Despite significant advances in scientific understanding, disease-modifying therapies remain critically lacking, underscoring the need for coordinated efforts across the HD community to accelerate therapeutic development.

At the forefront of this work is Critical Path Institute's (C-Path) Huntington's Disease Regulatory Science Consortium (HD-RSC). HD-RSC is C-Path's public-private partnership dedicated to advancing regulatory science tools and strategies to support more efficient and impactful HD drug development. HD-RSC brings together a diverse group of stakeholders, including people living with HD and their families, nonprofit research and advocacy organizations, clinicians, academic researchers, industry partners, and regulatory agencies, all working toward a shared mission: accelerating the development of therapies that meaningfully improve the lives of those affected by HD.

This year, HD-RSC is proud to announce the kickoff of the Lived Experience Working Group (LEWG), a new initiative designed to directly engage people living with HD and representatives from grassroots HD advocacy organizations that are members of HD-RSC. The LEWG is a dedicated forum for individuals impacted by HD to share their experiences, concerns, and hopes for the future while also providing opportunities for HD-RSC leadership to share updates and gather feedback on ongoing regulatory science initiatives and drug development activities in the HD space.

Importantly, this bidirectional exchange of information will directly inform several key HD-RSC initiatives, including:

Engagement with regulators about clinical trial endpoints in early-stage HD

Imaging biomarker efforts evaluating volumetric magnetic resonance imaging (vMRI) measurements of the caudate and putamen as candidate prognostic biomarkers for HD clinical trials (to be submitted to the FDA's Biomarker Qualification Program)

New collaborations exploring the use of digital health technologies (DHTs) in early-stage HD

The LEWG reflects a growing recognition across the rare disease and regulatory science landscape that meaningful therapeutic development cannot occur without close engagement with the lived experience community. Ensuring that clinical trial designs, endpoints, technologies, and therapeutic goals align with what matters most to people living with HD is essential to developing therapies that are not only scientifically robust, but also feasible, relevant, and meaningful.

Advocacy organizations participating in HD-RSC, along with the community members they invite to participate, will help shape and guide the direction of the LEWG. The group will prioritize impact over activity, focusing on tangible contributions that strengthen HD drug development and amplify patient and caregiver perspectives throughout the process.

“We appreciate this continued effort to bridge the gap between the scientific and regulatory communities with young people impacted by HD,” said Jenna Heilman, Executive Director of the Huntington’s Disease Youth Organization and member of HD-RSC and the Lived Experience Working Group. “Young people are often overlooked, but their experiences are invaluable to better understand the true impacts of Huntington’s Disease, especially as research is adapting to focus on earlier disease progression.”

As we recognize Huntington’s Disease Awareness Month, we are reminded that progress depends on partnership, transparency, and shared purpose. Through initiatives like the LEWG, HD-RSC continues to advance a collaborative model that places the lived experience community at the center of innovation and regulatory science.

Together, we move closer to a future where effective therapies for Huntington’s disease become a reality.

For more information on HD-RSC please visit: Huntington’s Disease Regulatory Science Consortium