# C-Path Concludes 2025 Global Impact Conference with Progress Across Rare Diseases, Neurology and Pediatrics

*Global regulators, industry leaders, scientists, and patient advocates set near-term commitments on early interception, trial modernization, and patient-first evidence.*

**WASHINGTON**, Sept. 11, 2025 — Critical Path Institute® (C-Path) today announced the outcomes of its Global Impact Conference (CGIC), held September 9–11 in Washington, D.C. Over three days of working sessions, regulators, industry leaders, researchers, clinicians, and patient advocates outlined priority actions and next steps to further strengthen collaborations that accelerate drug development across rare diseases, neurology, type-1 diabetes, and pediatrics — with the voices of those with lived experience playing a critical role in shaping the discussions.

Each day opened with a powerful keynote dialogue that set the tone and priorities and closed with tangible commitments—turning discussion into action and ensuring momentum carried forward.


Day 1, the opening plenary, **Accelerating Therapies for Disease Modification and Interception: Removing Barriers, Realizing Impact**, focused on strategies to accelerate the development of therapies for earlier stages of disease, and ways to reduce delays in development. The session on **Development of Gene-Based Therapies in Pediatrics** addressed critical considerations around trial design, safety, and equitable access for children and families. Breakout discussions on Implementing **New Approach Methodologies in Drug Development: Use Cases and What's Still Needed** highlighted practical applications and identified the tools, data, and standards required to support future regulatory decisions.

Day 2 focused on modernizing generating evidence that is both patient-centered and actionable. The opening session, **Modernizing Clinical Trials: Practical Innovations to Optimize Evidence Generation**, explored approaches to transform the entire evidence generation enterprise, to optimize human-centric translation, make trials more efficient and relevant, including selecting endpoints that matter to patients, integrating digital measures that function seamlessly at clinical sites, and improving data quality while reducing burden. **Putting People First: Patient-Focused Drug Development** brought the lived experience into the conversation, emphasizing its role in shaping study design, consent processes, and long-term follow-up. A global spotlight session, **Global Evidence in Medicine–Parkinson's Disease: Women as the Missing Voice in Parkinson's**, outlined specific steps to close long-standing data gaps and strengthen representation in research protocols and analyses.

Soania Mathur, M.D., shared a moving poem about her experience as a woman living with Parkinson's, emphasizing the need to spark a movement toward research that investigates sex-based differences underlying the disease. She underscored the critical importance of generating high-quality evidence to identify reliable, sex-specific biomarkers and social determinants of health, as well as to inform the development of tailored treatments and clinical guidelines.

C-Path is uniquely positioned to help drive this shift, having curated more than 15,000 patient data records for women with Parkinson's disease. This growing evidence base serves as a powerful resource to advance individualized and precision medicine approaches—ensuring that research reflects real patient experiences and translates into care strategies that improve quality of life for women and, ultimately, the entire Parkinson's community.

Day 3 placed advocacy and real-world evidence at the center of the conversation. **Today's Voices, Tomorrow's Treatments: The Growing Role of Patient Advocacy in Innovation** highlighted how coalitions succeed when priorities are aligned, decisions are transparent, and credit is shared across partners. **Real-World Data for Drug Development** examined strategies to generate decision-ready evidence from real settings while minimizing unnecessary complexity for participants and clinical sites.


"CGIC is about turning dialogue into action and outcomes," said Klaus Romero, M.D., M.S., FCP, Chief Executive Officer of C-Path. "We come away from CGIC with clear priorities, defined actions, and the collaborations needed to carry them forward. For the individuals and families we serve, every day matters. Our job is to make those days count."

Regulators were integral to the discussions throughout the Global Impact Conference, reinforcing the importance of generating evidence that drives meaningful decisions. They emphasized that patients cannot afford wasted time, underscoring the need for measures that truly matter to patients, study designs that work in real-world clinical settings, and proposals that are clear in purpose, proportionate in risk, and built to deliver learning quickly. By focusing on the right questions, regulators stressed, the field can move treatments to the people who need them faster.

Individuals with lived experience brought a critical perspective to the week's sessions.

"What we are starting to realize is that discovery-science really needs the patient experience. We don't have a lot of people that look like me as patient advocates or in clinical trials," said Glenda V. Roberts, Mount Sinai Health System. "We need to make a stronger effort to make sure that there are more diverse people represented. I'm talking about the lived experiences, different age groups, different experiences for rich genetic biological information that we need to understand."

Industry and academic partners stressed the importance of alignment. "Before we're executing trials and finalizing protocols, we're taking steps to really look at who the patient is to make sure they are reflected in the trials," said LaShell Robinson, Takeda. "I know that at times people think this is slowing down the trial, but if you think about it logically starting where the patient is and trying to make those connections, engaging with patient advocacy to understand the different patient journeys, actually speeds up trials."

Full session recordings will be published on C-Path's YouTube channel. To be notified when videos are available, subscribe to C-Path news and select CGIC updates at c-path.org/subscribe.

**About Critical Path Institute**

Founded in 2005, as a public-private partnership in response to the FDA's Critical Path Initiative, Critical Path Institute® (C-Path) celebrates its 20th anniversary as a vital, independent, nonprofit. C-Path's mission is to lead collaborations that advance better treatments for people worldwide. Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Path's global headquarters is located in Tucson, Arizona and C-Path's Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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