CRITICAL PATH INSTITUTE

2025 C-Path Global Impact Conference in Washington D.C. a Huge Success

We're excited to host **the 2025 Critical Path Institute® (C-Path) Global Impact Conference (CGIC), September 9–11**, at the Washington Marriott at Metro Center in downtown Washington, D.C. is under way!

Building on the momentum of CGIC 2024, this year's event brings together leaders and innovators from across the life sciences ecosystem to explore how C-Path's collaborative programs are driving progress in neurology, rare diseases, pediatrics, and beyond.

This year marks C-Path's 20th anniversary, making CGIC 2025 a particularly meaningful occasion. Join us to connect the dots across our growing portfolio and discover how C-Path can support your goals in advancing medical innovation and improving patient outcomes worldwide.

Whether you've joined us from **industry, regulatory agencies, academia, patient organizations, or as someone with lived experience,** you'll find valuable insights, thought-provoking discussions, and meaningful opportunities to collaborate.

Day 1 of Critical Path Institute's Global Impact Conference, **#CGIC2025**, opened with powerful conversations on accelerating therapies, amplifying patient voices, and strengthening public-private partnerships (PPPs) to address the most pressing needs in pediatrics and rare diseases.

We began with **Accelerating Therapies for Disease Modification and Interception: Removing Barriers, Realizing Impact**, moderated by Klaus Romero (C-Path).

Panelists from the FDA, EMA, Sanofi, and a patient organization explored how Public-Private Partnerships can bridge discovery with clinical application across the human lifespan. The discussion underscored opportunities to align regulators, enable earlier interventions, and overcome operational and financial barriers to developing therapies for at-risk populations.

"My daughter died and I want to give as much meaning to her life as possible," said Julia Vitarello, founder of Mila's Miracle Foundation. "Let's use privacy ethics to help us offer more treatments, better treatments, and not create another barrier to why millions of children can't be treated. Every single parent I've talked to, hundreds around the world, would all be very happy to know that their children's lives were meaningful in helping the next child, and that means sharing every little bit of pre-clinical data possible."

Next, in **Ensuring a Focus on Patients in Pediatrics and Rare Disease Therapy Development**, patient advocates and researchers highlighted strategies for designing trials that work for children and families. From understanding socioeconomic barriers to providing better support systems, the conversation emphasized how meaningful patient engagement can drive participation and shape the future of rare disease research.

The late morning session, **Development of Gene-Based Therapies in Pediatrics**, addressed the promise and challenges of regenerative treatments for young individuals. Panelists explored patient, disease, and regulatory factors, while also sharing strategies to ease the burden of trial participation for families.

During the lunch break, attendees joined three interactive breakout sessions:

- **Implementing New Approach Methodologies (NAMs) in Drug Development: Use Cases and What's Still Needed**, showcased the promise of human-relevant tools and the need for validation, standardization, and collaboration to advance their use in regulatory decision-making.
- **RDCA-DAP Taskforces: A Novel Small-scale Public-Private Partnership Approach for Rare Disease** highlighted how targeted, data-driven collaborations can accelerate regulatory-grade solutions for rare diseases.
- **What Happens After Approval? Rethinking Long-Term Follow-Up in Pediatric Gene Therapy** explored innovative approaches to post-approval monitoring that balance scientific rigor with the realities of pediatric care.

In the afternoon, **The Missing Voices in Neonatal Drug Development: Harnessing Patient Advocacy for Impact**, highlighted the lived experiences of families navigating neonatal care. Their stories, combined with perspectives from advocacy leaders and regulators, emphasized the urgency of bringing the patient voice into neonatal drug development. This was followed by Mechanism-based Drug Development for Rare and Orphan Diseases That Affect Pediatric and Adult Populations, which explored how target and disease considerations shape study design and highlighted unmet needs in developing new treatments.

We closed with a **Call to Action: Looking to the Future of Public-Private Partnerships for Pediatrics and Rare Diseases**, where panelists reflected on Day 1 themes and discussed how to optimize global collaborations across disciplines. The conversation reinforced a central idea: PPPs remain vital to translating science into solutions for patients.

Day 1 reminded us that progress depends on the convergence of science, regulation, and patient experience — and together, we are building the foundation for a healthier future.

C-Path's Global Impact Conference | Every Stage, Every Step: Transforming Lives Through Optimized Drug Development | Day 2 Recap

Day 2 of the C-Path Global Impact Conference built on the momentum of opening day, bringing together leaders, advocates, and researchers to explore how patient voices and innovative partnerships are transforming drug development.

We began with a deep dive into modernizing the entire evidence generation enterprise, where speakers underscored how adaptive trial designs, real-world data, and human-centric translation are reshaping the future of actionable evidence generation.

"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 the trial."

"When I paint with a broad brush on what's working in industry and what's not working in industry, what's working is C-Path, and C-Path is not congratulated enough on this intersection — the quality of the regulatory science that has happened very quietly is extraordinary," said Amitabh Chandra (Harvard Business School).

The conversation continued with a **powerful patient-focused drug development panel**, centered on the lived experiences of patients, caregivers, and advocates in shaping more compassionate, effective treatments.

The late morning session, **Connecting the Dots: Disease Interception in Parkinson's and Type 1 Diabetes** , highlighted surprising parallels between the two diseases and the opportunities for cross-disease learning to advance early intervention.

At midday, attendees explored three breakout sessions running in parallel:

- **Applying FDA's PFDD Roadmap to Digital Measures in Rare Neurodegenerative Diseases** – showcasing how patient input and regulatory alignment guide innovative outcome assessments.
- **C-Path's Disease Model Coalition** – rethinking traditional approaches to accelerate therapy development across therapeutic areas.

- **Critical Path Data and Analytics Platform (CP-DAP) Demo** – a hands-on look at C-Path's secure, collaborative platform advancing regulatory-grade research.

The afternoon continued with sessions on greater equity in Parkinson's research through the Global Evidence in Medicine-Parkinson's Disease initiative, where patient advocates and scientists emphasized the urgent need to amplify women's voices in clinical trials, and a compelling discussion on surrogate endpoints in PKD and Alzheimer's.

We closed the day with a forward-looking panel on public-private partnerships and early disease interception, underscoring the transformative potential of collaboration across sectors.

Day 2 left us inspired by the ways those with lived experience, regulators, researchers and industry are coming together to reimagine what's possible.