

C-Path Concludes 2026 Global Impact Conference with a Shared Agenda for Rebuilding Evidence Generation

Regulators, industry leaders, scientists, and those with lived experience converged on how evidence is generated, interpreted, and acted upon across rare diseases, neurology, and pediatrics.

TUCSON, Ariz., September 22, 2026 — Critical Path Institute® (C-Path) today announced the outcomes of its 2026 Global Impact Conference (CGIC), held September 15 and 16 at the Washington Marriott at Metro Center in Washington, D.C. Across two days of plenary sessions, breakout discussions and fireside conversations, regulators, industry leaders, researchers, clinicians and advocates, joined by people living with the conditions under discussion, addressed what must change in how evidence is generated for drug development in the 21st century.

Programming was organized around the conference theme: **Evidence Driven. Patient Focused. Future Ready.** Sessions examined the methods that generate actionable evidence, the mechanisms that bring patient experience into regulatory decision-making, and the technologies shaping the next decade of drug development.

Day One

C-Path Chief Executive Officer Klaus Romero, M.D., M.S., FCP, opened the conference with Michael Davis, M.D., Director of the Center for Drug Evaluation and Research at the U.S. Food and Drug Administration, in *A New Evidence Frontier*, a plenary session on why rare diseases, where large-scale randomized controlled trials are frequently not feasible, require a different approach to generating evidence. The agenda then moved to the decisions made at the outset of drug development. Fixing the Front End of Drug Development examined target selection as an under-recognized component of the evidence-generation continuum, one that shapes biomarker strategy and trial feasibility long before first-in-human studies.



“More than ever with the amount of data that we’re collecting — genomic sequencing, biomarkers, multi-omics, along with the use of artificial intelligence — it gives us additional tools necessary to define the population and patient,” Davis said.

In speaking about digital health technologies, Davis added, “In terms of evidence collection, this data can be a lot richer than conducting measures at a clinic visit, so we’re looking at what really matters most to patients: how are they doing in their day-to-day lives?”

Breakout sessions covered C-Path’s Digital Health Technology Thesaurus, demonstrated by C-Path scientists Grace Lee, Ph.D., and Fraser Bocell, Ph.D., and an assessment of artificial intelligence and real-world data in rare muscular dystrophies led by Ramona Belfiore-Oshan, Ph.D., of C-Path with Andre Paredes, Ph.D., of the Muscular Dystrophy Association. Afternoon sessions addressed digitally derived endpoints in chronic heart failure, neonatal drug development, and the conditions under which AI produces evidence regulators can use. A closing plenary took up post-approval evidence and sustained patient access.

Day Two

Day two opened with a fireside chat between C-Path board member Milad Alucozai, DrPH, general partner at Pamir Ventures and C-Path CEO Klaus Romero. The two spoke about emerging technologies and where the next generation of development tools will originate. Nicholas King, M.S., executive director of C-Path’s Predictive Safety Testing Consortium, and Gary Friedman, M.D., clinical co-chair of its Biomarker Data Repository, followed with a session on repurposing biomarkers across safety and disease applications.



Breakout sessions examined New Approach Methodologies qualification against the FDA’s recent roadmap and guidance documents, and presented progress from the Rare Disease Cures Accelerator-Data and Analytics Platform on a clinical trial simulation tool for Friedrich’s ataxia now under FDA review. Later discussions covered consent and governance in data sharing for individualized therapies, neuroimaging biomarkers as a route toward biologically grounded disease staging, and how patient input reaches regulatory decision-

makers. Romero closed the conference with Michelle Campbell, Ph.D., of the FDA with a panel about the next evolution of collaborations built to optimize evidence generation.

Lived Experience at the Center

Each day reserved a place in the main program for lived experience presentations.

On day one, Adith Thummalapalli spoke about how the healthcare system serves adults living with a condition long treated as pediatric. Thummalapalli is president of the Adult Advisory Committee at Parent Project Muscular Dystrophy, which represents the teen and adult voices of the Duchenne and Becker community, and has held patient advocate roles with the FDA, the Department of Defense, academic institutions, and industry sponsors.

On day two, patient advocate Jessica Morris shared her experience of living with SOD1-associated amyotrophic lateral sclerosis (ALS). Her perspective reinforced why those with lived experience have a meaningful role in shaping drug development.

“I want you to leave this room understanding that behind every clinical endpoint, every trial participant and every data point is a person who is fighting to preserve a life that they love and that patient experience should help shape how we measure success, design trials and develop treatments,” Morris said.



Fireside Conversations



Three fireside discussions paired Romero and C-Path Chief Data and Technology Officer Christopher Lunt with leaders from across the ecosystem. Billy Amzal, Ph.D., of the Gates Foundation and Lynne Yao of the FDA discussed the growing role of disease modeling, model-informed drug development, and integrated data in improving decision-making. Amit Khanna, Ph.D., of Novartis Pharmaceuticals and Julia Vitarello with Mila's Miracle Foundation addressed scaling personalized medicine

through collaborative innovation, and Janet Woodcock, M.D., former principal deputy commissioner of the FDA, discussed Data's over-and-under-use, examining which analytical approaches change outcomes and which add complexity without insight.

“Translational science is underfunded and it’s totally disproportionate,” Woodcock said. “So that means the data (translational) are not valued at the level they should be, and there hasn’t been the investment necessary in the industry.”

“What we have now is nonprofits doing this...they’re collecting the data on their patients, they’re setting up registries, they’re trying to collect biological data because no one else is doing it.”



“CGIC exists because the problems in front of this field cannot be solved by any single organization,” said Romero. “The work presented over two days came out of consortia rather than any single laboratory or sponsor: a clinical trial simulation tool for Friedreich’s ataxia now under FDA review; a terminology resource bringing common language to digital health measurement; qualification pathways for new approach methodologies; and biological staging frameworks maturing in parallel across Alzheimer’s, Parkinson’s, Huntington’s, and ALS. For the individuals and families waiting on these therapies, the work does not end when the conference does.”

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](https://www.fda.gov/cpi), 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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