CRITICAL PATH
INSTITUTE

Advancing Drug Development. Improving Lives. Together.

March 31, 2026

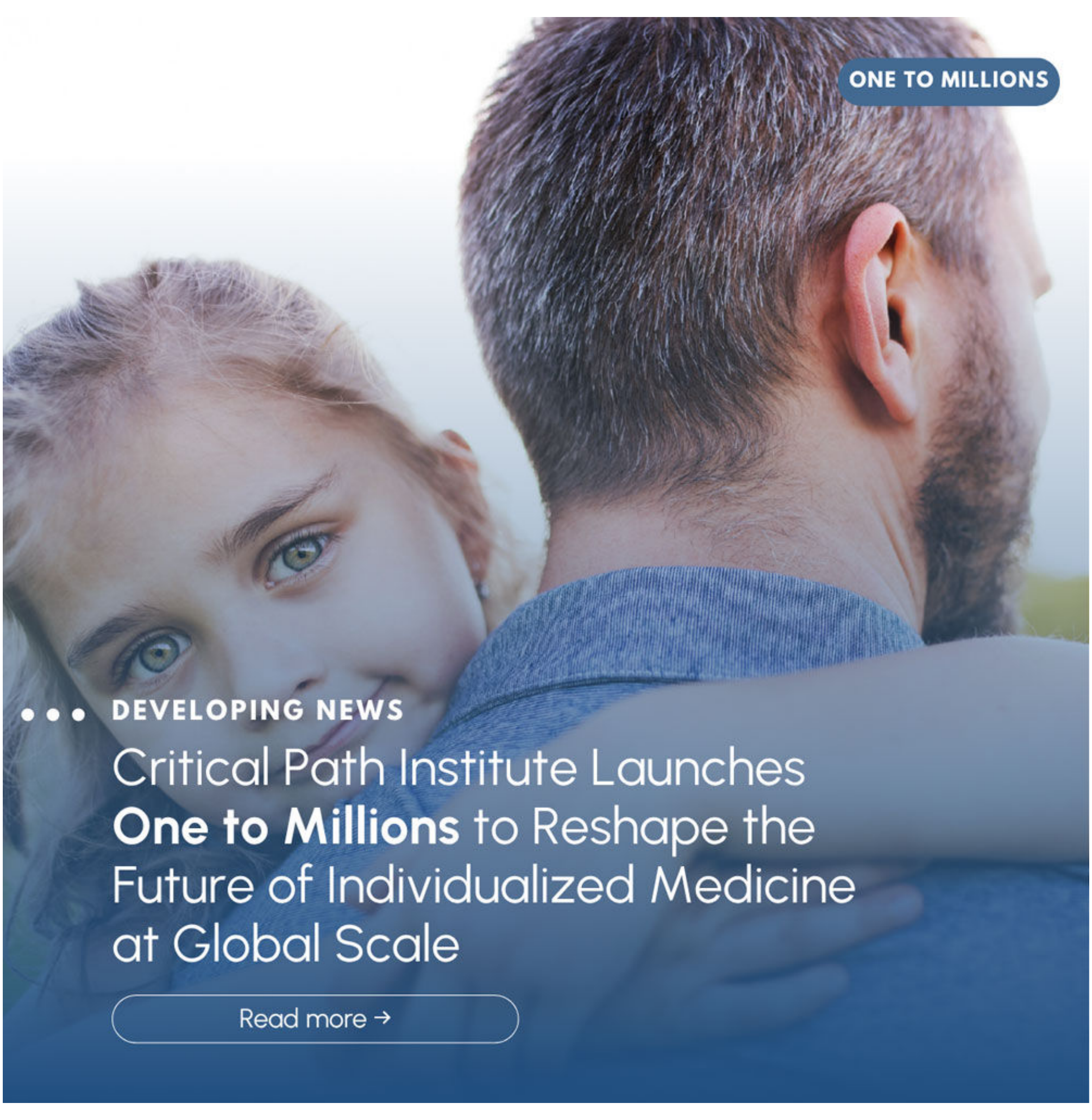
C-Path RARE/ORPHAN AND PEDIATRIC DISEASE PROGRAMS: QUARTERLY NEWSLETTER

Welcome to the Rare/Orphan and Pediatric Disease Programs quarterly newsletter. In between major announcements, webinars and meetings, this communication serves to update you on the latest developments within C-Path's rare and pediatrics programs, as well as the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP). None of these advancements are possible without the participation of our members, collaborators and data contributors. Thank you.

As spring 2026 begins and conference season gets underway, we're pleased to share updates from our team. February marked Rare Disease Awareness Month, culminating in Rare Disease Day on February 28—a time to amplify the voices of more than 300 million people worldwide living with a rare disease, along with their families. C-Path remains proud to lead collaborative efforts that accelerate drug development for these communities.

In March, we welcome new team members and prepare for upcoming webinars and conferences. We look forward to the opportunities ahead. Read on for the latest updates and what's on the horizon in the coming months.

SPOTLIGHT: One to Millions Launch and Welcome Megan Miller



On Thursday, March 26, Critical Path Institute announced the launch of **One to Millions**, a new global, public-private initiative focused on moving highly individualized therapies from breakthrough science to real, timely access for patients. **One to Millions** brings together patients, families, advocacy leaders, regulators, and industry partners to align how we evaluate and deliver these highly personalized therapies, so that promising treatments don't stall before reaching the people who need them most.

You can read the full announcement here and join the conversation on Wednesday, **April 8, 12 p.m. ET** for a live discussion on Scalable Approaches to Patient-Centered Benefit–Risk: Defining Patient-Focused Drug Development in Precision Medicine.

Welcome Megan Miller, CP-RND Director of Outreach


Megan Miller

A neurobiologist by training, Megan brings more than a decade of experience working at the intersection of science, policy, and community engagement to build partnerships that accelerate research and improve health outcomes. She joins C-Path from the National Institute on Aging, where she led engagements with federal and international partners, advocacy organizations, and the U.S. Congress. Megan also brings experience leading community-centered disease advocacy and advisory initiatives, building close relationships with individuals and families affected by rare neurodegenerative diseases and advancing the integration of lived experience into research. Earlier in her career, she managed international research collaborations at USAID, working closely with communities and stakeholders to drive scientific progress and evidence-informed decision-making.

RDCA-DAP UPDATES

The platform currently hosts data for over 44 disease areas, including the largest database for Friedreich's ataxia. For a full list of diseases and platform engagement to date, visit Additional RDCA-DAP Resources.

NORD CORNER

Attend the NORD Rare Disease Scientific Symposium

The agenda is now live for the 2nd annual NORD Rare Disease Scientific Symposium, happening this April 14-15 in Arlington, VA, and virtually. You won't want to miss this event full of insights you won't get anywhere else about rare disease clinical research, trial design, and translational science. Explore the agenda, which includes a keynote address from NIH Director Dr. Jay Bhattacharya, and secure your tickets today.

Earn Your Stripes: Rare Disease CME Challenge

1 in 10 Americans lives with a rare disease, but medical education on them is sorely lacking. On Rare Disease Day, rare patients showed their stripes to raise awareness for their conditions. Now, it's time for doctors to step up and earn their stripes too. Pediatricians, primary care physicians, and specialists all have a role to play in diagnosing, referring, and treating rare patients. Take the Rare Disease CME Challenge by finishing at least one free, accredited education course by March 31.

Visit medlive.com/nord to get started.

WEBINAR SERIES 2026

Thursday, April 9, 12 PM ET | Register now for our upcoming webinar, 'Hope on the Horizon for ADTKD Patients'

On Demand Webinars

March 19: Meeting Patients Where They Are: Validating Remote Digital Tools for Multi-Indication NMD Research.

In this session, Dr. Duong explores the scientific, clinical, and regulatory considerations involved in validating video-based assessments and other digital tools across multiple NMD indications. Through both presentation and panel discussion, attendees gained valuable insights into how these approaches can enhance accessibility for patients regardless of location, reduce the burden of participation in clinical trials, generate reliable, regulatory grade data, ensure innovation remains grounded in patient-centered research, and more.

***You can view all 2025 ROPD Webinars on demand here.**

For more information about Rare/Orphan and Pediatric Disease Programs, visit: https://c-path.org/area-of-focus/rare-and-orphan-diseases/.

ANNOUNCEMENTS AND ADDITIONAL RESOURCES

Save the Date, C-Path's Global Impact Conference is set for Sept. 15-16, in Washington, D.C.

C-PATH
2026
GLOBAL IMPACT CONFERENCE
WASHINGTON, DC | SEPT 15-16

March 17: PBC Ireland Partners with C-Path's Rare Disease Data Platform, Advancing Research Efforts for Primary Biliary Cholangitis.

February 28: The Critical Path Institute Podcast: Connecting the Dots in Rare and Pediatric Diseases with VP Collin Hovinga.

February 26: Amidst Rare Disease Awareness Month, C-Path Shines a Light on Listening As a Form of Precision Medicine.

February 12: Building the Infrastructure of Hope.

February 1: Turning the Impossible into the POSSIBLE.

December 12: Emerging Therapies in Autosomal Dominant Polycystic Kidney Disease.

Help support our mission. MAKE A GIFT TODAY

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FDA Acknowledgment