CRITICAL PATH INSTITUTE

Turning the Impossible into the POSSIBLE

February is Rare Disease Month. It is a time when the global community unites to support the 350 million people worldwide living with a rare disease.

The urgency to find answers is clear. But so is the path forward.

Time is too precious to waste on restating the problems of the past. Everyone knows working in silos doesn't lead to success. The weakness of fragmentation is well established. We need to talk about the strength of collaboration.

Critical Path Institute (C-Path) is the place where that collaboration happens.

Recognized as a trusted, neutral convener, we are bringing people together to synergize for the greater good. We are bringing regulators into the process early so everyone is clear on what evidence is needed to support regulatory approval and patient access. This purpose is expressed through our own Rare/Orphan and Pediatrics Disease Program. We convene stakeholders (the regulators, the industry leaders, the researchers, the people with lived experience, and the patient organizations) to reach data-driven consensus on what success looks like, and to help foster and create the solutions needed to achieve it.

This is how progress scales.

When stakeholders decide early what outcomes matter, it saves valuable time. Through C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform, we help partners integrate and standardize patient-level data. This allows researchers to quantify disease progression with a level of accuracy that was previously thought impossible.

Working alongside this data engine is the Rare Disease Clinical Outcome Assessment Consortium; the team that defines the standards for clinical trials so that we know if a drug is effective. With these universal standards in place, we can tackle the unique challenges of individual disorders.

But data is only powerful when it reflects the lived experience of each person affected.

During a panel at C-Path's Global Impact Conference last September, Andrew Rosen, CEO of the National Ataxia Foundation, highlighted exactly why that matters. He noted that for years, clinical assumptions missed the mark. But when researchers collaborated directly with patients, they realized the most urgent reality of the disease was unmanaged pain. Andrew's story proves that listening is a form of precision medicine. Both C-Path's Duchenne and Huntington's disease regulatory science consortia operate on this exact principle. We are taking the daily reality of these diseases and translating it into the evidence regulators require.

For other conditions, the challenge is proving a therapy is working with biomarkers sensitive enough to detect change. The teams at C-Path are dedicated to finding these answers. In every instance, our goal is to clear the regulatory path so that safe drugs can reach the people who need them without delay.

The field moves faster when we move together. That is the work C-Path is here to support.

C-Path is proud to stand with the families, researchers, and regulators who are building this future. Together, we are turning the impossible into the possible. And we are using this month to show you exactly how that happens.

Follow C-Path on LinkedIn throughout Rare Disease Month to see the roadmap for yourself.