

What Collaboration Makes Possible

Based on the CGIC 2025 Panel, [A Call to Action from Day 1: A look at the future of public-private partnerships for pediatric and rare diseases.](#)

On a Tuesday afternoon in the fall of 2019, a biostatistician from a pharmaceutical company, a division director from the FDA, a pediatric neurologist running a natural history study in Germany, and a mother who had spent six years tracking her daughter's seizure patterns in a spreadsheet sat down at the same table. None of them would have been in the same room without C-Path. Each of them had a piece of the puzzle that the others needed. And over the next several hours, they did something that sounds simple but is extraordinarily difficult in practice: they agreed on how to measure whether a therapy is actually helping the children it was designed for.

Agreeing on measurement is where rare disease drug development either begins or stalls. A company can have a promising molecule; a regulatory agency can have the authority to approve it; families can have the willingness to enroll their children in a trial. But if no one has established what progression looks like in the disease, or what counts as a meaningful improvement, or how to capture that improvement in a way that satisfies regulators on multiple continents, no amount of promising science can move it forward.

C-Path has been convening exactly these kinds of groups, in one disease area after another, for more than twenty years. The data collaboration, the qualified biomarkers, the methods, the outcome measures, and the regulatory relationships that emerged from that collaboration are reshaping how therapies can reach patients in a timely manner.

A patient registry for a single rare pediatric condition requires negotiating data-sharing agreements with clinical sites in a dozen countries, harmonizing the way each site records symptoms and milestones, and collecting enough longitudinal data to establish how the disease actually behaves over time. Once accessible to research communities, it gives every subsequent researcher a shared foundation that didn't exist before. Study designers know what variability looks like. Families can see that the endpoints in a trial reflect their actual experience of the disease and regulators know what natural decline looks like from a patient centric perspective.

Biomarker qualification is another layer of that collaborative foundation. Companies that compete in every other therapeutic area need a neutral space where their scientists can sit together with regulators and academic investigators, pooling data and analysis to determine which biological signals reliably track with disease activity. C-Path provides that space. Once a biomarker is qualified through this collaborative process, it becomes a shared resource available to any developer. A company designing a pivotal trial can use it without spending years establishing its validity from scratch. A regulator reviewing the results can trust the measurement. Months and sometimes years are knocked off the development and review timelines, and the benefit flows directly to those with lived experience.

Pediatric extrapolation may be the most consequential example of what this kind of sustained collaboration produces for integration of innovation as part of global development. For decades, whether adult efficacy data could be leveraged to support pediatric approvals was a question that different regulatory agencies answered differently, sometimes for the same disease. Regulatory Agencies across the globe have now agreed on common approaches to integrate extrapolation methods in pediatric development. C-Path and its partners bring regulators, industry scientists, patients and clinical investigators together to build a

systematic structure for advancing extrapolation methods based on underlying biology rather than procedural habit. Developers can design focused pediatric studies that address genuine uncertainties rather than repeating what adult trials have already demonstrated. Approved therapies get to children sooner, and the studies designed ask sharper, more meaningful questions removing burden on children and their families.

Neonatal pharmacology has long posed its own version of the same challenge. Estimating safe and effective doses for the youngest patients has always been constrained by their physiological complexity and the ethical weight of enrolling them in conventional dose-finding studies. Physiologically based pharmacokinetic models, developed through collaborative research and refined through regulatory engagement, are now generating dose estimates that require only minimal clinical confirmation. A field that once had to choose between impractical trials and educated guesswork is finding a third option.

Global regulatory alignment has historically been one of the most stubborn barriers in pediatric rare disease development. A company might design a study that satisfies one agency's expectations only to learn, years later, that another agency needed something different. The shared frameworks, qualified tools, and collaborative standards that C-Path and its partners have developed over the past two decades are helping to reduce those gaps. When regulators across agencies are working from the same disease understanding, the same validated biomarkers, and the same body of natural history evidence, the path forward for a developer looks more consistent from one region to the next. That consistency means fewer redundant studies, faster timelines, and less burden on children and their families.

A new generation of therapies is arriving that will draw on every layer of this infrastructure. Gene therapies designed for small patient populations. Gene editing approaches tailored to individual mutations. Treatments so personalized that the traditional randomized controlled trial may need to evolve into something new. Evaluating these therapies will require new kinds of evidence, new regulatory thinking, and new forms of global collaboration. And the community that has spent over twenty years learning how to do exactly that, learning how to sit in a room together, learning how to reconcile competing priorities, and learning how to build shared tools that none of them could build alone, is ready.

Somewhere in the next year, a biostatistician, a regulator, a gene therapy developer, and a parent will sit down at the same table to work out how to evaluate a therapy unlike anything the regulatory system has seen before. What they agree on will help the next family, and hundreds to thousands after that, get access to treatments that would otherwise never clear the regulatory path. C-Path will have put them in the same room.

During Rare Disease Awareness Month, the more than twenty years of work that made that room possible is the best reason to believe that the era of leaving rare disease patients and their families behind has come to an end.

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