
Advancing Rare Disease Innovation Through Responsible Data Sharing — Key Takeaways from the RISE Together: Data Sharing Across the Rare Disease Ecosystem



From L-R: C-Path VP of Rare/Orphan and Pediatric Disease Programs Collin Hovinga, Director of Outreach Megan Miller, CEO Klaus Romero, Executive Director of C-Path's Duchenne Regulatory Science Consortium Ramona Belfiore-Oshan, and Vice President of Strategic Partnerships Jennifer Kendra.

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At a recent public workshop co-convened by the Duke-Margolis Institute for Health Policy and the FDA Rare Disease Innovation Hub, leaders from across the rare disease ecosystem gathered to address a critical challenge: how to responsibly share data to accelerate therapeutic development and regulatory decision-making.

C-Path was proud to join regulators, researchers, patient advocates, and industry stakeholders in this important dialogue, bringing its deep expertise in data collaboration to the forefront of the conversation.

The Urgency of Data Sharing in Rare Diseases

Rare diseases present unique scientific and operational challenges. Small, heterogeneous patient populations often limit the availability of robust datasets, making it difficult to fully understand disease progression or design efficient clinical trials.

Throughout the workshop, participants emphasized that **high-quality, shareable data is essential** to overcoming these barriers. When effectively leveraged, shared data can:

- Inform disease progression models
- Support the selection of meaningful clinical endpoints
- Improve clinical trial design
- Strengthen safety monitoring frameworks

Ultimately, these advances can help bring treatments to patients faster, an urgent priority for the rare disease community.

C-Path's Contributions to the Dialogue

C-Path played an active role across multiple sessions, contributing both thought leadership and real-world experience:

- **C-Path CEO Klaus Romero** moderated a session exploring the key components and stakeholders involved in successful data sharing ecosystems
- **VP of Rare/Orphan and Pediatric Disease Programs Collin Hovinga** shared practical examples demonstrating how data sharing is already being implemented to drive impact
- **Duchenne Regulatory Science Consortium Executive Director Ramona Belfiore-Oshan** and **C-Path Board Member Ron Bartek** participated in a panel discussion on building the infrastructure and tools needed to enable scalable, sustainable data sharing

These contributions reflect C-Path's long-standing commitment to advancing collaborative, data-driven approaches to drug development, particularly in rare diseases, where maximizing the value of limited data is essential.

Aligning with the One to Millions Initiative

The themes of the workshop closely align with C-Path's **One to Millions** initiative, which aims to transform how data is generated, shared, and utilized across the drug development lifecycle.

By enabling insights to scale from individual patients to broader populations, One to Millions seeks to unlock new efficiencies in advancing drug development, helping ensure that no patient's data is underutilized and no opportunity for learning is missed.

Looking Ahead

The discussions reinforced a clear and compelling message: **collaboration and responsible data sharing are foundational to the future of rare disease innovation.**

As stakeholders continue to align on standards, infrastructure, and best practices, initiatives like this workshop, and organizations like C-Path, will play a pivotal role in accelerating progress.

Together, the community is charting a path toward faster, more effective delivery of therapies for patients living with rare diseases.

You can view the recording and materials from this workshop, [here](#).

Want to learn more about C-Path's efforts in Rare and Orphan Diseases?

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