CRITICAL PATH INSTITUTE

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

In this episode of the Critical Path Institute© Podcast, C-Path Vice President of Rare/Orphan and Pediatric Disease Programs Collin Hovinga, joins host and CEO Klaus Romero to discuss the current landscape of rare diseases, the unique challenges and opportunities they present, and how C-Path is uniquely positioned to accelerate drug development for these conditions. This podcast highlights how rare diseases collectively represent a vast and complex group with about 10,000 known rare conditions and numerous subtypes. These differences present challenges, but also opportunities due to shared learnings and in some instance similar underlying disease mechanisms.

C-Path's approach to advancing drug development centers on collaboration among regulators, industry, patient advocacy groups, and researchers, ensuring that patient voices are integral to drug development. A key resource in this ecosystem is the Rare Disease Cures Accelerator-Data and Analytics Platform, a hub that aggregates, standardizes, and curates' diverse data sources such as registries, clinical trials, and natural history studies. Through the reuse of data, the platform avoids redundant research, supports innovative clinical trial designs, and facilitates regulatory decision-making by providing tools such as trial simulations and alternative control models.

The conversation highlights examples of successful applications of these tools in diseases like Friedreich's ataxia, polycystic kidney disease, and Duchenne muscular dystrophy, emphasizing the importance of regulatory engagement and public-private partnerships. The podcast also covers the legislative framework supporting rare neurodegenerative diseases, specifically the ACT for ALS, which fosters collaboration between FDA, NIH, C-Path, patients, and industry to accelerate drug development.

Highlights

- Introduction to the Rare Disease Cures Accelerator-Data and Analytics Platform, C-Path's data platform that aggregates and standardizes rare disease data to accelerate drug development.
- Description of trial simulation tools and how they support regulatory decisions and clinical trial design.
- Importance of regulatory engagement and public-private partnerships in successful rare disease drug development.
- Discussion of the Act for ALS and how it fosters collaboration among NIH, FDA, industry, patients, and C-Path.
- Insight into pharmaceutical industry participation and trust in C-Path's role as the neutral convener.

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