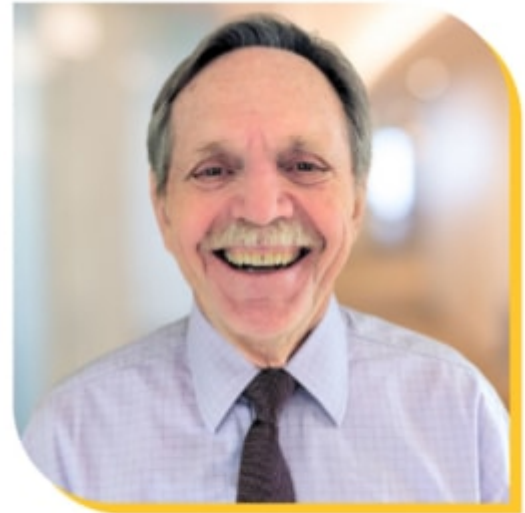


The Critical Path Institute Podcast: Accelerating Drug Development in Friedreich's Ataxia, with Ron Bartek

The newest Critical Path Institute® (C-Path) podcast features Ron Bartek, president and co-founder of the Friedreich's Ataxia Research Alliance (FARA), for an insightful conversation with C-Path CEO and podcast host Klaus Romero.

Ron shares the inspiring journey of how FARA was established nearly 28 years ago after his son was diagnosed with Friedreich's ataxia (FA), a rare neurodegenerative disease with no approved treatments or substantial research at the time. Faced with a void in scientific and clinical data, Ron, his wife and a small community of parents, patients, and scientists collaborated to create a research alliance that would foster collaboration and accelerate drug development for FA.



Ronald J. Bartek

This conversation highlights the critical early efforts needed to develop clinical outcome measures and natural history data, which became foundational for clinical trials, regulatory submissions, and eventual drug approval. There became an urgent need to develop a natural history database, which collected longitudinal data from patients examined by clinicians following standardized protocols.

This database was integrated into C-Path's FDA-funded Rare Disease Cures Accelerator Data and Analytics Platform (RDCA-DAP®), enabling data sharing, analysis, and regulatory-grade evidence generation. This neutral, high-quality data platform supported the approval of Omaveloxolone, the first FDA-approved drug for FA, marking a watershed moment for the rare disease community.

Key Insights:

- Ron's story underscores how patient families can drive research innovation when institutional interest and funding are absent. By mobilizing scientists, clinicians, and families, FARA created a collaborative ecosystem that transformed zero data into actionable clinical research.
- The episode highlights that the foundational role of developing clinical outcome measures tailored to FA by establishing reliable, standardized endpoints is essential before clinical trials can begin, especially in diseases lacking precedent.
- The strategy of having multiple clinicians worldwide conduct uniform assessments and enter data electronically ensured high data quality and comparability. This natural history study became a critical resource, both as a historical comparator in trials and as a continuous data source for understanding disease progression, embodying best practices for natural history studies in rare diseases.