

C-Path Announces First Data Transfer from Takeda to Accelerate Alpha-1 Innovation

TUCSON, Ariz., May 6, 2025 — Critical Path Institute's® (C-Path) Critical Path for Alpha-1 (CPA-1) consortium is proud to announce the receipt of its first data contribution in efforts to accelerate innovative therapies for alpha-1 antitrypsin deficiency (AATD). The phase 2 clinical trial dataset originates from Takeda, a founding member of the CPA-1 consortium.

“This contribution represents a pivotal step for CPA-1,” expressed Collin Hovinga, Pharm. D., Vice President of Rare/Orphan and Pediatric Disease Programs at C-Path. “The CPA-1 consortium was formed to assist in the acceleration of the development of AATD therapies by integrating data into C-Path's Rare Diseases Cures Accelerator–Data Analytics Platform (RDCA-DAP®) and leveraging it to design regulatory-grade drug development tools. We are grateful to Takeda for not only this dataset contribution but also their support for the critical role data sharing may play in potentially advancing treatments for rare diseases.”

AATD is a genetic disorder impacting approximately 1 in 2,500-3,000 individuals globally that frequently leads to liver and lung damage, including fibrosis. Current therapeutic approaches—such as liver transplant reserved for end-stage liver disease, or alpha-1 antitrypsin augmentation therapy for lung associated disease—do not target the root cause of AATD, underscoring the need for greater disease management options.

“Significant unmet medical needs in AATD persist because of gaps in understanding, particularly concerning liver disease,” said Nirav Desai, M.D., Executive Medical Director and Fazirsiran (TAK-999) Global Program Leader, Rare Gastrointestinal and Inflammation Therapeutic Unit, at Takeda. “By contributing our AATD datasets and other analyses to the CPA-1 platform, we aim to foster greater collaboration and information exchange, helping to drive much-needed advancement in the care of this underdiagnosed progressive disease.”

As a leading values-based, R&D-driven biopharmaceutical company, Takeda brings decades of experience in gastroenterology and rare immunology to the CPA-1 consortium, as well as a deep understanding of the very real needs of the AATD patient community. To build on this first dataset transfer, Takeda will endeavor to provide additional datasets that could contribute to the understanding of natural history and clinical trial protocol design.

Organizations interested in learning more about the CPA-1 consortium, or contributing data to RDCA-DAP, can email cpa1admin@c-path.org or visit c-path.org/rdca-dap.

About Critical Path Institute

Founded in 2005, as a public-private partnership in response to the FDA's [Critical Path Initiative](#), Critical Path Institute® (C-Path) celebrates its 20th anniversary as a vital, independent, nonprofit. **C-Path's mission is to lead collaborations that advance better treatments for people worldwide.** Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Path's global headquarters is

located in Tucson, Arizona and C-Path's Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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