AIRNA Joins C-Path's Alpha-1 Antitrypsin Deficiency Consortium to Advance Innovative AATD Treatments

AIRNA, a clinical-stage company developing RNA-editing medicines with an initial focus on AATD, joins C-Path's antitrypsin consortium upon initiation of its RepAIR1 global clinical study.

TUCSON, Ariz., April 22, 2026 — Critical Path Institute's® (C-Path) Critical Path for Alpha-1 Antitrypsin Deficiency Consortium today announced the addition of AIRNA as its newest industry partner, bringing RNA editing expertise to the consortium's efforts to accelerate drug development for alpha-1 antitrypsin deficiency (AATD). AIRNA joins existing industry members CSL Behring, Sanofi, Takeda, and Beam Therapeutics, as well as a coalition of academic clinician researchers, patient advocacy groups, and regulatory colleagues working collaboratively to advance regulatory-endorsed drug development solutions for patients living with AATD.

Genetic approaches to therapeutic development for AATD, a disease caused by identified mutations in the gene that codes a key protease inhibitor, are entering and moving through clinical trials. RNA editing, gene editing, and other novel modalities are demonstrating the potential to address the underlying genetic cause of AATD rather than managing symptoms alone. These advances bring specific regulatory challenges around endpoints, biomarkers, and clinical trial design, making precompetitive collaboration through public-private partnerships such as C-Path's alpha-1 consortium essential to ensuring that new therapies reach patients as efficiently and safely as possible.

AIRNA is developing a new class of RNA-editing medicines with the goal of delivering functional cures for people with rare and common diseases, beginning with AATD. Its lead program, AIR-001, is designed to address the underlying cause of AATD. By correcting the disease-causing mutation at the RNA level without permanently altering DNA, AIRNA aims to combine the precision of genetic medicine with the flexibility of a reversible, repeatable treatment approach delivered subcutaneously. AIR-001 has been granted Orphan Drug Designation for the treatment of AATD from the U.S. Food and Drug Administration.

AIRNA's addition to the consortium brings an important perspective as the AATD therapeutic landscape continues to expand with diverse genetic approaches. "At C-Path, we believe that breakthroughs in rare and pediatric disease happen when the best minds and the most innovative partners come together around a shared mission, and we're proud to welcome AIRNA as a contributor to our consortium's efforts in endpoints and biomarkers," said Collin Hovinga, Pharm.D., Vice President of Rare/Orphan and Pediatric Disease Programs at C-Path. "AIRNA brings exactly the kind of scientific ingenuity we need to move faster for patients and families counting on us, and we're excited to advance that work together."

"We are proud to partner with C-Path and the AATD community as we advance a potential best-in-class RNA-editing therapy for people living with AATD," said Jacob S. Elkins, M.D., Chief Medical Officer of AIRNA. "For patients to benefit from new treatment approaches in AATD, the field needs strong collaboration across patients, researchers, industry and regulators to improve how therapies are developed and evaluated."

The consortium's mission is to forge actionable, regulatory-endorsed drug development solutions for patients living with AATD by convening key stakeholders in a public-private partnership dedicated to open collaboration. Alpha-1 consortium members bring data, individual and organizational expertise, and a desire for change to hepatic and pulmonary workgroup sessions. The consortium actively integrates data from clinical trials, natural history studies, and registries through C-Path's Rare Diseases Cures Accelerator–Data Analytics Platform, supporting the development of novel drug development tools and optimizing patient-centric clinical outcomes focusing on biomarkers and regulatory-grade solutions that reflect the growing role of biologic and genetic therapies in the AATD treatment landscape.

As new treatment modalities move through the pipeline, C-Path's alpha-1 program will continue working to ensure that the endpoints, biomarkers, and clinical trial standards needed to evaluate them are in place, advancing the shared goal of better outcomes for patients and families affected by this rare disease.

Organizations interested in learning more or contributing data to C-Path's Rare Disease Cures Accelerator can visit c-path.org or email cpa1@c-path.org.

About Critical Path Institute
Critical Path Institute (C-Path) is an independent, nonprofit established in 2005 as a public-private partnership in response to the FDA's Critical Path Initiative. 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 .

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the Department of Health and Human Services (HHS) and is 56% funded by the FDA/HHS, totaling $23,740,424, and 44% funded by non-government source(s), totaling $18,881,611. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement by, FDA/HHS or the U.S. Government.

About AIRNA
AIRNA is a clinical-stage company developing RNA-editing medicines designed to repair disease-causing genetic variants and introduce beneficial variants that promote human health. The company's proprietary platform is based on research by RNA-editing pioneers and co-founders Thorsten Stafforst (University of Tübingen) and Jin Billy Li (Stanford University). AIRNA is advancing a pipeline of RNA-editing therapeutics led by a product candidate for alpha-1 antitrypsin deficiency (AATD). AIRNA is headquartered in Cambridge, MA, with research operations in Tübingen, Germany. Learn more at https://airna.com/.

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