

C-Path Consortium Adds Gene Editing and Advanced Imaging Leaders to Accelerate Alpha-1 Research

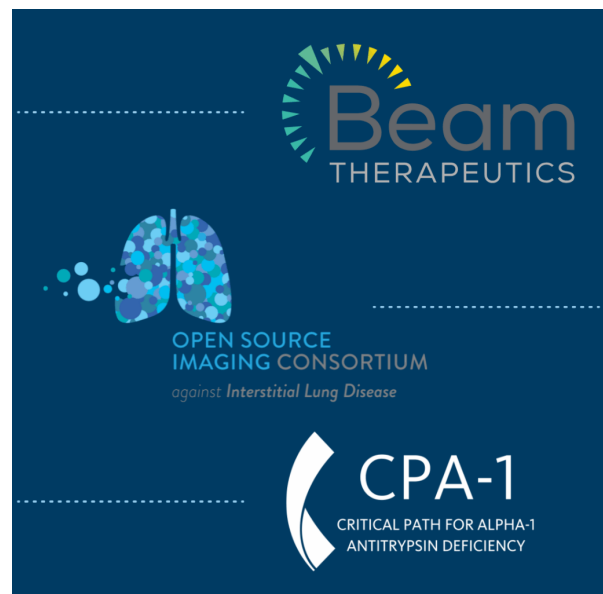
Beam Therapeutics and the Open Source Imaging Consortium join C-Path's CPA-1 program, bringing gene editing innovation and global lung imaging data resources to advance new therapies for alpha-1 antitrypsin deficiency.

TUCSON, Ariz., Nov. 24, 2025 — Critical Path Institute® (C-Path) today announced that its Critical Path for Alpha-1 Antitrypsin Deficiency (CPA-1) consortium is expanding with the addition of Beam Therapeutics and the Open Source Imaging Consortium (OSIC). Together, the new members bring expertise in the development of genetic medicines and world-leading lung-imaging data science, respectively. By joining CPA-1, Beam Therapeutics and OSIC will collaborate on the consortium's aim to accelerate the development of Alpha-1 antitrypsin deficiency (AATD) therapies.

AATD, a genetic condition affecting more than 3 million people worldwide, has no cure and presents significant challenges for diagnosis and drug development. There are approximately 100,000 people in the U.S. with the most common severe genotype, PiZZ, of which only 10% are diagnosed. Its diverse clinical presentation and variable disease progression create challenges for patients, clinicians and regulators. By integrating novel genomic strategies with advanced imaging analytics, CPA-1 aims to reduce that uncertainty and strengthen the scientific foundation needed to accelerate transformative therapies.

In AATD, a mutation in the SERPINA-1 gene leads to the misfolding and abnormal processing of a key protease inhibitor. As a result, the protein cannot reach the lungs to protect against tissue damage, and the protein accumulates in the liver where it causes liver damage. Intravenous augmentation therapy is currently the only approved treatment for lung manifestations of the disease but requires weekly intravenous infusion and slows down lung damage but does not prevent it. There are no approved therapies for liver disease at this time, and patients are managed with supportive care or in severe cases with liver transplantation. As a result, there is an unmet need for novel therapies that can address both the lung and liver manifestations of patients with AATD.

“Individuals and families affected by AATD deserve the fastest and safest path to innovative treatments,” said Collin Hovinga, Pharm.D., Vice President of Rare/Orphan and Pediatric Disease Programs at C-Path. “Beam Therapeutics’ leadership in gene editing and OSIC’s global lung-imaging expertise reflects exactly the type of cutting-edge, collaborative capabilities that will propel CPA-1’s work forward. Their participation strengthens the consortium’s ability to reduce uncertainty in AATD drug development and move promising solutions closer to patients.”



“A biorepository for alpha-1 antitrypsin deficiency can be transformative — it can drive better science, diagnostics, and treatments,” said Elizabeth Estes, Executive Director of OSIC. “And it’s only possible through real collaboration. OSIC is proud to be part of this work with C-Path on behalf of patients everywhere.”

Advances in molecular technologies have the potential to reshape the therapeutic landscape for genetic diseases, and Beam Therapeutics’ precision base-editing platform offers the potential to address the root cause of AATD by correcting the genetic mutation, thereby potentially enabling treatment of both the lung and liver manifestations of AATD through a single course of treatment. As gene editing and other genetic medicines continue to move forward in clinical trials, Beam’s expertise provides critical insight into the scientific, clinical and regulatory considerations needed to safely and effectively advance these approaches.

“At Beam, we are motivated by the unmet need of patients living with AATD to create a new class of single-course, durable treatments that can address the full spectrum of disease manifestations,” said Amy Simon, M.D., chief medical officer at Beam. “Building on our existing relationship with C-Path, this partnership with the CPA-1 program is an important step to collaborating with key stakeholders in the alpha-1 community in our collective effort to enable and accelerate the development of novel innovative treatments in AATD.”

At the same time, monitoring lung pathology and disease progression remains central to diagnosing AATD and evaluating emerging therapies. OSIC’s leadership in standardizing lung-imaging data, combined with its open-science model, gives CPA-1 the ability to explore imaging biomarkers and data standards that may support more consistent clinical assessment and more efficient regulatory review. OSIC, a global 501(c)(3) consortium of academic, industry and patient-advocacy partners, has curated thousands of imaging datasets worldwide and continues to build a comprehensive, globally representative repository. Its collaborative, open-source approach supports the development of imaging-based tools for diagnosis, prognosis and prediction of treatment response.

With the complementary strengths of Beam and OSIC, CPA-1 is positioned to bring genomic innovation and advanced imaging science together in a way that can meaningfully accelerate progress for the AATD community.

Visit [CPA-1](#), [Beam Therapeutics](#) and [OSIC](#) to learn more.

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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