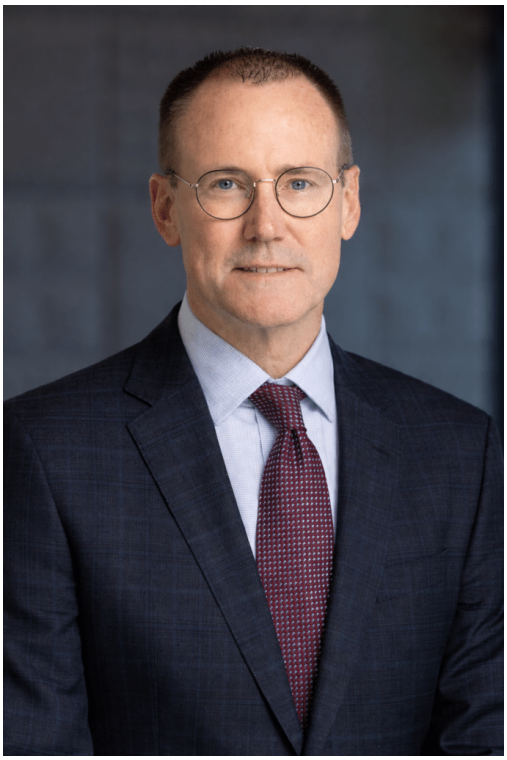
CRITICAL PATH INSTITUTE

Redefining Endpoints Through Lived Experience: Jon Hagstrom's Story of Patient Advocacy in Alpha-1 Antitrypsin Deficiency



Founded in 2023 by Critical Path Institute (C-Path), Critical Path for Alpha-1 Antitrypsin Deficiency was convened to address an urgent need: accelerating meaningful medical progress for people living with Alpha-1 Antitrypsin Deficiency (AATD). C-Path's Critical Path for Alpha-1 Antitrypsin Deficiency brings together industry partners, clinician scientists, regulators, and—critically—patients, working side by side to advance better tools, endpoints, and therapies for AATD.

From the outset, collaboration with patient groups has been central to Critical Path for Alpha-1 Antitrypsin Deficiency's mission. The consortium was designed not only to build consensus for drug development tools that advance regulatory decision-making, but also to ensure that patient experience informs every step of the scientific and regulatory pathway for both liver and lung targeted therapies.

One of the patient advocates driving this work is Jon Hagstrom, whose journey with Alpha-1 underscores why patient voices matter so deeply. Jon was diagnosed with Alpha-1 at age 39, after years of unexplained shortness of breath that persisted even after he quit smoking. When he sought care from a pulmonologist in New York City, his symptoms initially resembled more common respiratory conditions—but his young age and severely diminished lung function raised red flags.

"By the time I was diagnosed, my lung function was already down to 29 percent. That's when you realize how critical early detection really is," Jon reflected.

Testing confirmed a diagnosis of Alpha-1, which quickly triggered the need for augmentation therapy to replace Jon's missing Alpha-1 protein in an effort to slow his disease progression. However, as Jon explained, the treatment is most effective when started early, before significant lung function is lost, because this therapy cannot restore damaged lung tissue. Despite treatment, his disease continued to progress. Within five years, he was undergoing an evaluation for a lung transplant.

Because of strict organ-matching requirements—and Jon's 6'3" height—he waited more than a year at Duke University Medical Center before suitable lungs became available. By the time of his transplant, his lung function had declined to just 14 percent. Jon spoke candidly about the realities of lung transplantation: the heightened risk of infection and rejection, the lifelong medical regimen, and the profound changes to daily life and long-term outlook.

"So by the time he [Jon's son] was four, I was on oxygen 24/7. I dragged an oxygen concentrator to his kindergarten orientation. He used to call it Daddy's Special Air. In his early days, I even hired a nanny though I was home."

Amid these challenges, Jon found the Alpha-1 Foundation, which became both a source of support and a

platform for advocacy. He began by attending conferences and participating in fundraising events. Over time, his commitment and perspective led to deeper involvement—first through the Foundation's Finance and Development Committees, and eventually as a board member and board chair.

That advocacy extends beyond the Foundation. Jon described how sustained engagement with regulatory leaders helped catalyze the creation of the CPA-1 pre-consortium at C-Path, where he now serves as a co-lead alongside Kristin Van Goor of Takeda. Together with the Alpha-1 Foundation and other stakeholders, he has worked to advance the development of meaningful clinical trial endpoints for Alpha-1 lung disease—ensuring that trials measure outcomes that truly matter to patients.

Jon's story reflects the core philosophy of C-Path and CPA-1: that progress in rare disease research is strongest when patients are not just participants, but partners. His living experience continues to inform the consortium's work, helping shape a future where earlier diagnosis, better trial design, and more effective treatments will be a new reality for the Alpha-1 community.

"Working with Jon is absolutely inspirational. His drive, intellect, hands-on approach and willingness to question are key for CPA-1's progress. It's an exciting time in this disease space as innovative therapies are both entering and wrapping up early clinical trials. The goal now of CPA-1 and its member community is to harness this momentum and deliver on our patient-focused, endpoint, and clinical trial tool programs," Sheryl Denker, Scientific Director, Critical Path for Alpha-1 Antitrypsin Deficiency.

To learn more about C-Path's CPA-1 Consortium click below:

Contact the CPA-1 Consortium