C-Path Welcomes New Advisory Members to Alpha-1 Antitrypsin Deficiency Consortium

TUCSON, Ariz., August 6, 2024 — Critical Path Institute's (C-Path) Critical Path for Alpha-1 Antitrypsin Deficiency (CPA-1) consortium today announced the addition of several key advisory members. The new members, recognized experts in their respective fields and patient advocacy organizations, will contribute their significant expertise to the consortium's mission to accelerate drug development for Alpha-1 Antitrypsin Deficiency (AATD), a rare genetic disorder.

Joining the consortium are:

- Alpha-1 Foundation
- COPD Foundation
- Global Liver Institute
- Jon Hagstrom, Board Member, Alpha-1 Foundation
- Noel G. McElvaney, Royal College of Surgeons in Ireland
- Marc Miravitlles, M.D., Pulmonologist at the Pneumology Department, University Hospital Vall d'Hebron / Vall d'Hebron Research Institute in Barcelona, Spain
- Jeff Teckman, M.D., Pediatric Hepatologist at Saint Louis University
- Alice Turner, MBChB, MRCP, PGCE, Ph.D., Professor of Respiratory Medicine, University of Birmingham, England
- Hugo E. Vargas, M.D., Professor of Medicine, Mayo Clinic
- Andrew A. Wilson, M.D., Professor of Medicine and Director of The Alpha-1 Center, Boston University and Boston Medical Center

"We're truly excited about the new members joining our consortium," said Amanda Klein, Pharm.D., Executive Director of the CPA-1 consortium. "With their deep expertise and fresh perspectives, they will be key in helping us develop new treatments more efficiently and effectively. This is a major step forward in our fight against AATD."

AATD affects the liver and lungs, leading to serious health conditions such as liver disease and emphysema. The disorder, impacting an estimated 100,000 people in the U.S. alone, often manifests as chronic obstructive pulmonary disease around 40 years of age. Infants with AATD also face significant risks, as approximately 10% exhibit liver function abnormalities that may eventually require transplantation.

The genetic complexity of AATD complicates our understanding of patient experiences and clinical trials. Recognizing these challenges, the CPA-1 consortium actively integrates data from clinical trials, natural history studies, and registries through C-Path's Rare Diseases Cures Accelerator–Data Analytics Platform (RDCA-DAP®). This collaborative effort supports the development of novel drug development tools and optimizes patient-centric clinical outcomes, focusing on biomarkers and regulatory-grade solutions to advance treatment options.

"The Alpha-1 Foundation is pleased to partner with the Critical Path Institute to advance the qualification of biomarkers," said Jon Hagstrom, Chair of Alpha-1 Foundation Board of Directors. "It is vital to the advancement of research and new therapies for Alpha-1 patients to identify new pathways. This partnership brings together all facets of the Alpha-1 community; research, academic, government, regulatory, and most

importantly, the voice of the patient community. As the Alpha-1 patient representative, I am proud to serve in this important collaboration and its success."

"The COPD Foundation welcomes the opportunity to partner with C-Path to advance the regulatory qualification of biomarkers in AATD lung disease," said Alan Hamilton, Ph.D., Senior Director of Research, PIVOT Lead, COPD Foundation. "In alignment with the Foundation's patient-centric PIVOT framework (Patient Inspired Validation of Outcome Tools), we enthusiastically support the inclusion of representatives from Patient Advocacy Organizations in this multi-stakeholder collaborative initiative. It is critical that discussions about outcome measures in AATD clinical trials are grounded in a deep understanding of the patients' lived experiences – the relationship between a candidate biomarker and how patients feel and function in their everyday life and/or survival will be a key consideration in assessing if the biomarker is fit-for-purpose in the context of regulatory decision-making."

"As both an Alpha One patient and an advocate, I am enthusiastic to be a part of a passionate group of stakeholders in the AATD community who share a common objective of leveraging data to amply the patient voice in drug development," shared Kristin Hatcher, Pediatric and Rare Liver Diseases Program Director at Global Liver Institute. "GLI is committed to enhancing the well-being of individuals and families affected by liver disease and solving the problems that matter to patients. I look forward to working with this consortium to develop ways to identify and incorporate patients' lived experiences to optimize clinical trial design and outcome measures."

"We are grateful for the expertise of our new members to push the boundaries of what's possible in AATD research and treatment," said C-Path's Pediatrics, CPA-1 Senior Scientific Director, Gina Smith, MPH, RN. "Their contributions will enable us to advance our understanding of the disease, improve clinical trial designs, and ultimately, improve outcomes for those living with AATD."