C-Path Launches Consortium for Alpha-1 Antitrypsin Deficiency

Critical Path Institute (C-Path) is excited to announce the launch of the Critical Path for Alpha-1 Antitrypsin Deficiency (CPA-1) Consortium. The CPA-1 consortium aims to accelerate drug development for alpha-1 antitrypsin deficiency (AATD), a rare disease that affects individuals and families worldwide. This will be achieved by integrating data through C-Path's Rare Diseases Cures Accelerator–Data Analytics Platform (RDCA-DAP®) and leveraging those data for CPA-1 to collaboratively develop regulatory-grade solutions to continuously address unmet needs in drug development for this condition, including:

- Generating evidence to support the use and regulatory acceptance of novel drug development tools, such as biomarkers, for AATD liver and lung disease clinical trials and;
- Identifying how to incorporate and utilize patients' lived experiences to optimize clinical trial design and outcome measures that capture how patients feel, function and thrive.

Through a grant provided by the U.S. Food and Drug Administration (FDA), C-Path has engaged stakeholders in a collaborative manner, identified areas of unmet need within drug development, potential solutions, and are evaluating available data to support a regulatory grade drug development tool for novel therapies to progress in both non-clinical and clinical development for AATD.

CPA-1 brings together industry, regulatory, academic, and AATD patient groups to find solutions to progress new therapies through the drug development process. The consortium is launching two work groups to focus efforts towards AATD-associated hepatic and pulmonary disease, which in the severest stages require liver or lung transplant. Currently, treatment options are limited for those living with AATD and there is a high unmet need to address the burdens of living with AATD through drug development efforts.

Inhibrx, Takeda and global biotherapeutics leader CSL Ltd have joined as founding members of the consortium signaling the launch of this collaborative effort.

"We are proud to be a founding member of this important collaborative effort and to apply Takeda's enduring expertise in gastroenterology to delineate a path to treatment options for people living with AATD. The burden of this rare disease is tremendous, and we are deeply committed to serving the community of patients and caregivers who for far too long have had to grapple with limited progress and innovation in the treatment landscape of this condition," said Obi Umeh, M.D., Vice President, Franchise Global Program Leader, Rare Gastrointestinal and Inflammation Therapeutic Area Unit at Takeda.

"Our membership underscores our promise to putting our profound knowledge and extensive experience in the research of rare respiratory diseases and the development of corresponding biotherapies for those whose lives are most directly impaired by them," said Lars Groenke, M.D., CSL's Vice President, Co-Lead R&D Respiratory Therapeutic Area. "We are convinced that with this tangible collaborative effort, we are shaping the future path forward in accelerating the development of, and access to, new treatment options for a community where there is unmet need."

The CPA-1 Consortium is the first C-Path effort to engage with the FDA's Center for Biologics Evaluation and Review (CBER). Celia Witten, M.D., deputy director of CBER, has been a guiding voice supporting the establishment of CPA-1. "There is a crucial need to develop more treatments for patients with AATD," Witten said. "There is currently no cure for this disease and no new treatments have been developed for AATD over the last three decades. This consortium will offer the opportunity for diverse stakeholders to

collaborate to develop tools for clinical development to accelerate progress in this area."

C-Path originated as an initiative to develop collaborative efforts with the FDA to address areas of unmet needs and through the years has developed a long track record of successes, particularly for rare diseases. For 20 years, C-Path has worked with the Center for Drug Evaluation and Research (CDER) to address some of the most challenging unmet needs within rare disease drug development. "We value the opportunity to once again collaborate with C-Path through the CPA-1 consortium that brings together our FDA colleagues from the hepatic and pulmonary review divisions and CBER," noted Jeffrey Seigel, M.D., Director, Offices of Drug Evaluation Sciences (ODES).

"We are excited by the opportunity to address a critical need and work with a passionate stakeholder group within the AATD community that shares a common goal that will bring solutions and accelerate the development of novel therapies for people living with AATD. The consortium starts from a strong place with years of academic and industry data to build a solution around AATD," commented Gina Smith, MPH, RN, C-Path Scientific Director for the CPA-1 consortium.

To learn more about C-Path's CPA-1 consortium and its pediatric program email cpa1@c-path.org.

CRITICAL PATH INSTITUTE

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.

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About CSL
CSL (ASX:CSL; USOTC:CSLLY) is a global biotechnology company with a dynamic portfolio of lifesaving medicines, including those that treat haemophilia and immune deficiencies, vaccines to prevent influenza, and therapies in iron deficiency and nephrology. Since our start in 1916, we have been driven by our promise to save lives using the latest technologies. Today, CSL – including our three businesses: CSL Behring, CSL Seqirus and CSL Vifor – provides lifesaving products to patients in more than 100 countries and employs 32,000 people. Our unique combination of commercial strength, R&D focus and operational excellence enables us to identify, develop and deliver innovations so our patients can live life to the fullest. For inspiring stories about the promise of biotechnology, visit CSL.com/Vita. For more information about CSL, visit CSL.com.

About Takeda

Takeda is focused on creating better health for people and a brighter future for the world. We aim to discover and deliver life-transforming treatments in our core therapeutic and business areas, including gastrointestinal and inflammation, rare diseases, plasma-derived therapies, oncology, neuroscience and vaccines. Together with our partners, we aim to improve the patient experience and advance a new frontier of treatment options through our dynamic and diverse pipeline. As a leading values-based, R&D-driven biopharmaceutical company headquartered in Japan, we are guided by our commitment to patients, our people and the planet. Our employees in approximately 80 countries and regions are driven by our purpose and are grounded in the values that have defined us for more than two centuries. For more information, visit www.takeda.com.

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