

C-Path to Lead Pre-Consortium Aimed at Transforming, Accelerating Medical Product Development in Lysosomal Diseases

TUCSON, Ariz., Sept. 1, 2022 — [Critical Path Institute \(C-Path\)](#) today announced the launch of a pre-consortium collaboration focused on accelerating medical product development in lysosomal diseases, supported by a grant from the U.S. Food and Drug Administration (FDA).

The pre-consortium will focus on defining tangible common unmet needs across lysosomal diseases, also referred to as lysosomal storage diseases, and laying the groundwork for specific solutions to these needs, which are envisioned to be generated by a future consortium.

C-Path's proven proficiencies will be leveraged to ensure the success of this effort, specifically the organization's track record in generating tangible solutions that have accelerated drug development in rare and pediatric indications, together with the abilities of the [Rare Disease Cures Accelerator-Data and Analytics Platform](#) (RDCA-DAP®) to integrate multiple patient-level data sources. This collaborative pre-consortium will provide a neutral environment for industry, regulatory agencies, academia, and the patient community to come together and actively contribute to advance promising solutions to facilitate medical product development for the benefit of patients and their families.

“We are working with the Critical Path Institute to launch a pre-consortium collaboration focused on accelerating drug development in lysosomal diseases,” said Center for Drug Evaluation and Research (CDER) director, Patrizia Cavazzoni, M.D. “We believe this effort will complement the work C-Path is already doing within the RDCA-DAP initiative, as well as other rare disease collaborative consortia efforts at C-Path.” The Lysosomal Diseases Pre-Consortium will be under the umbrella of CDER's [Accelerating Rare disease Cures Program](#).

Lysosomal diseases are inherited metabolic diseases that are characterized by an abnormal build-up of various toxic materials in the body's cells, as a result of enzyme deficiencies. Several individual conditions are part of this group of indications, and they may affect different parts of the body, including the skeleton, skin, heart, and central nervous system. There is an urgent need to accelerate the development of treatments for these conditions, and collaboration is essential to meet this challenge.

“C-Path is uniquely positioned to lead this new lysosomal pre-consortium,” explained Klaus Romero, M.D., M.S., F.C.P., C-Path's Chief Science Officer and Executive Director of Clinical Pharmacology. “We thank the FDA for recognizing the importance of collaborative projects and we look forward to leveraging our core competencies to provide strategic and tactical guidance, engage relevant stakeholders and bring diverse expertise to generate the solutions needed to address this unmet need.”

The Institute's [core competencies](#), which include expertise in data management standards, biomarkers, modeling and analytics, regulatory science, and clinical outcome assessments, help de-risk decision making in the development and regulatory review process of novel medical products.

To learn more and inquire about joining C-Path's lysosomal diseases pre-consortium, contact cpld@c-path.org.



About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit organization established in 2005 as a public and private partnership. C-Path's mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established numerous global consortia that currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and hundreds of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona, [C-Path in Europe](#) is headquartered in Amsterdam, Netherlands and [C-Path Ltd.](#) operates from Dublin, Ireland with additional staff in multiple other locations. For more information, visit c-path.org.

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