

## C-Path Launches Consortium to Accelerate Medical Product Development in Sickle Cell Disease



## September is National Sickle Cell Awareness Month

TUCSON, Ariz., Sept. 17, 2020 — The Critical Path Institute (C-Path) today announced the launch of the Critical Path for Sickle Cell Disease (CP-SCD) Consortium to support collaboration and regulatory endorsement of new medical product development tools for sickle cell disease. These tools will help to optimize and de-risk clinical trials to increase efficiency in developing and delivering safe, effective treatments for people living with sickle cell disease.

September is National Sickle Cell Disease Awareness Month. SCD affects between 90,000 and 100,000 people in the U.S., and millions more around the world. The disease is caused by genetic mutations that cause red blood cells to become deformed or sickled, which can result in anemia. Additionally, the deformed red blood cells can obstruct blood flow, causing recurrent pain, organ damage and even early death. Sickle cell disease research was neglected for many years and while research in this field has now accelerated, currently approved therapies for the disease are not curative.

"The sharing and integration of knowledge, data and collaboration across different organizations is key in our efforts to generate tools that will accelerate medical product development for sickle cell disease," said C-Path Chief Science Officer Klaus Romero, M.D., M.S., F.C.P. "C-Path is uniquely positioned to lead CP-SCD as it has extensive experience in accessing and leveraging data from people living with rare diseases to support regulatory-grade solutions for unmet needs in medical product development."

Research in SCD has been limited, but a renewed interest in the disease and novel technologies has resulted in many potential therapeutics in development, as well as recent drug approvals. However, the path to regulatory approval of these new therapeutics is challenged by limited understanding of the natural history of the disease and how to measure therapeutic effects on the different aspects of sickle cell disease. CP-SCD has been set up to drive a comprehensive understanding of these issues, and to develop tools to overcome them.

"A goal of this consortium is to establish an international forum that engages regulatory agencies around the globe to identify and address issues that impact the development and approval of new therapies for sickle cell disease," said CP-SCD Executive Director, Jane Larkindale, D.Phil. "We are excited to announce this new consortium during Sickle Cell Awareness Month and continue to work towards making a difference in the sickle cell community."

Visit <a href="https://c-path.org/programs/cp-scd/">https://c-path.org/programs/cp-scd/</a> for more information. Institutions, organizations and individuals interested in collaborating with CP-SCD should email: <a href="mailto:jlarkindale@c-path.org">jlarkindale@c-path.org</a>

## **About C-Path:**



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 dozens of pharmaceutical and biotech companies. C-Path US is headquartered in Tucson, Arizona and C-Path, Ltd. EU is headquartered in Dublin, Ireland, with additional staff in multiple other locations. For more information, visit www.c-path.org and c-path.eu.

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