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Doris Duke Charitable Foundation Awards Grant to Critical Path Institute to Advance Therapies for Sickle Cell Disease

Critical Path Institute (C-Path) Will Plan a Consortium of Stakeholders Focused on Developing Therapies for Sickle Cell Disease

NEW YORK, September 6, 2018 — In a continuing effort to spur advancements in treating and curing sickle cell anemia, the Doris Duke Charitable Foundation today announced a grant to the Critical Path Institute (C-Path) to lay the groundwork for forming a collaboration of multiple stakeholder organizations to accelerate the development of therapies for the disease. With the funding, C-Path will conduct the planning necessary to bring together scientists, clinicians and representatives from the biopharmaceutical industry, governmental regulatory agencies, academic institutions and patient advocacy organizations to identify and work collaboratively toward solving significant challenges affecting the entire research and development community. The anticipated outcome is a consortium model engaging potential partners to work on jointly beneficial drug development tools and novel methodologies, submit them to the regulatory agencies for endorsement, and then make them available to the broader research community.

This announcement coincides with National Sickle Cell Awareness Month, which began in September 1983 to foster public awareness about the genetic disease. Sickle cell disease is the most common inherited blood disorder in the United States, affecting an estimated 90,000 to 100,000 Americans, according to researchers. Those with the disease often suffer from chronic pain and crippling side effects beginning as early as childhood and, in severe cases, face a reduction in life expectancy of 20 to 30 years compared to those without the disease.

"With new drugs and approaches such as gene editing in development, the sickle cell disease research community urgently needs agreed-upon endpoints for clinical trials to decide if a therapy is working," said Betsy Myers, program director for medical research at the Doris Duke Charitable Foundation. "We are excited to support C-Path in launching this effort of coordinating many important stakeholders, each of whom play unique, critical roles in reaching the ultimate

goal of providing sickle cell disease patients with important therapies, and even cures. C-Path is in an excellent position to organize this effort given its public-private partnership with the Food and Drug Administration and established role of building consortia that speed the development of new medical therapies for patients who suffer from a wide range of diseases and conditions."

"C-Path plays a key role in providing the strategic and scientific direction that drives successful drug development consortia and spurs progress," said Lynn Hudson, Ph.D., chief science officer at C-Path. "We are grateful to the DDCF for this funding, which will allow us to assemble a team of dedicated stakeholders united in identifying the best and most promising treatments for sickle cell disease. Sickle cell research is of special interest to the foundation and it's with a shared commitment and clear objectives that we will collaborate to meet the urgent need for better treatment outcomes."

About the Organizations:

C-Path (Critical Path Institute) 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 over 1,500 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and dozens of pharmaceutical and biotech companies. C-Path is headquartered in Tucson, Arizona, with additional staff in multiple remote locations. For more information, visit <u>www.c-path.org</u>.

The mission of the Doris Duke Charitable Foundation is to improve the quality of people's lives through grants supporting the performing arts, environmental conservation, child well-being and medical research, and through preservation of the cultural and environmental legacy of Doris Duke's properties. The foundation's Medical Research Program supports clinical research that advances the translation of biomedical discoveries into new preventions, diagnoses and treatments for human diseases. The foundation has supported sickle cell disease research through a variety of grant-making mechanisms, including the <u>Sickle Cell Disease/Advancing</u> <u>Cures</u> awards, which were launched in 2017.