Nemours Children's Health to Host First Gene Therapy Clinical Trial for Morquio A Syndrome

WILMINGTON, Del. (May 16, 2023)– As part of the Foundation for the National Institutes of Health (FNIH) Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium (AMP® BGTC), Nemours Children's Health has been chosen to conduct a first-of-its-kind gene therapy clinical trial for Morquio A syndrome.

Nemours Children's will work collaboratively with the FNIH AMP® BGTC, a public-private partnership between the National Institutes of Health (NIH), U.S. Food and Drug Administration (FDA), biopharmaceutical and life science companies, and non-profit and other organizations, to help speed the development and delivery of customized or 'bespoke' gene therapies. Eight genetic diseases were selected for clinical trials, which will take place across the country. Nemours Children's was chosen for their Morquio A project proposal and will also be the site of the clinical trial.

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Nemours Children's Health is internationally renowned for diagnosing and treating Morquio A syndrome, a rare skeletal dysplasia, affecting 1 in 200,000 births, caused by an inherited gene mutation. Leading pediatric orthopedic surgeons and geneticists at Nemours have established a nationally recognized skeletal dysplasia program. Dr. Tomatsu has held NIH grant funding for Morquio syndrome since 2010. Furthermore in 2021, additional NIH funding was established to develop a complete natural history for Morquio A syndrome. Nemours researchers have also been studying new and innovative treatments to help cure this genetic disorder that causes serious complications including cervical spinal cord compression, short stature, flat feet, difficulty walking, tracheal obstruction, hearing loss, and heart valvular disease.

Most recently, Dr. Tomatsu and his colleagues identified through murine models that Adeno-associated virus (AAV) gene therapy could be the answer for these patients. With AAV gene therapy, patients are given a one-time infusion which consists of a viral vector containing the correct gene sequence that can transfer genetic material safely into tissues and cells.

"The ultimate goal of our work is to help our patients. With the knowledge we gain during this trial, we believe Nemours Children's Health will be able to offer Morquio patients the most up-to-date and effective therapies available," said Stuart Mackenzie, MD, Orthopedic Surgeon, Director of the Skeletal Dysplasia Clinic, Nemours Children's Health, Delaware. "Through this specialized public and private partnership with FNIH AMP, we are able to help realize our vision to create the healthiest generations of children."

For the remainder of the year, FNIH and Nemours will focus on refining the treatment protocol, planning, and hiring additional professional staff. Nemours Children's aims to start enrollment for the clinical trial in 2024.

Nemours Children's would also like to acknowledge the many other colleagues that have been instrumental in developing this clinical trial including Michael B. Bober, MD, Medical Director of Orthogenetics, Kimberly Kipner, Clinical Research Coordinator, Allison Bradford, Clinical Research Assistant and Lan He, Research Lab Manager.

Private philanthropy in support of novel research efforts such as this is critically important. There have been several generous individuals and families who have provided funding throughout the course of this work. In particular, Nemours would like to acknowledge the dedicated fundraising efforts of *A Cure for Robert, Inc. (Rooting for Robert)* and the generous support of the Angelo R. Cali & Mary V. Cali Family Foundation, Inc. and Morquio Community, Inc.

About Nemours Children's Health

Nemours Children's Health is one of the nation's largest multistate pediatric health systems, which includes two free-standing children's hospitals and a network of more than 70 primary and specialty care practices. Nemours Children's seeks to transform the health of children by adopting a holistic health model that utilizes innovative, safe, and high-quality care, while also caring for the health of the whole child beyond medicine. Nemours Children's also powers the world's most-visited website for information on the health of children and teens, Nemours KidsHealth.org.

The Nemours Foundation, established through the legacy and philanthropy of Alfred I. duPont, provides pediatric clinical care, research, education, advocacy, and prevention programs to the children, families and communities it serves. For more information, visit Nemours.org.

About The Foundation for the National Institutes of Health

The Foundation for the National Institutes of Health (FNIH) connects the world's leading public and private organizations to accelerate biomedical breakthroughs for patients, regardless of who they are, where they live, or what disease they have. Together with leading scientists and problem-solvers, and a successful track record of navigating complex problems, the FNIH accelerates new therapies, diagnostics, and potential cures; advances global health and equity in care; and celebrates and trains the next generation of scientists. Established by Congress in 1990 to support the mission of the NIH, the FNIH is a not-for-profit 501(c)(3) charitable organization. For more information about the FNIH, please visit fnih.org.

About AMP®

AMP® BGTC is one of many AMP programs expediting discovery around Alzheimer's disease, Parkinson's disease, Schizophrenia, Rheumatoid Arthritis and Lupus, Type II Diabetes, Common Metabolic Diseases, Heart Failure and Autoimmune and Immune-Mediated Diseases, all coordinated by the FNIH since the 2014 launch of the large-scale initiative. The AMP partnerships use cutting-edge scientific approaches to bring new medicines to patients by enhancing validation of novel, clinically relevant therapeutic targets and biomarkers. To learn more about AMP, visit https://fnih.org/AMP.

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