

Scenic Biotech Enters Cooperative Research & Development Agreement with the National Institutes of Health for Genetic Modifier-Based Treatment Approach for Niemann-Pick Type C Disease

CRADA combines Scenic Biotech's genetic modifier platform with NIH's patient registry to unlock genetic modifiers as a path to novel therapies for Niemann-Pick Type C disease

Amsterdam, the Netherlands, February 01, 2023 – [Scenic Biotech](#), a pioneer in the discovery of genetic modifiers developing therapeutics to treat severe diseases, today announced that it has entered into a Cooperative Research and Development Agreement (CRADA) with the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD), part of the National Institutes of Health (NIH), to support its therapeutic program for the treatment of Niemann-Pick Type C (NPC) disease, which is a severe, progressive and lethal neurological disorder. The goal of the collaboration is to obtain clinical insights into genes that can reduce the severity of NPC. Under the terms of the CRADA, NICHD will grant Scenic Biotech access to its extensive clinical dataset and whole genome DNA sequence data derived from NPC patients. Forbes D. Porter, MD, PhD, Senior Investigator at NICHD, will be the principal investigator for this study based on his long-running clinical protocols that track the natural history of rare genetic disorders, including NPC.

"The CRADA with the NIH will help advance our work toward identifying genetic modifiers for NPC. Our aim is to develop novel therapeutics that act by suppressing or completely blocking the effect of a disease-causing mutated gene. Through the combined efforts with Dr. Porter's team, we hope to further enable the discovery of a tailor-made, disease-modifying small molecule therapeutic for this devastating disease," commented **Sebastian Nijman, CSO and co-founder of Scenic Biotech**.

About Scenic Biotech

Scenic Biotech is at the forefront of identifying genetic modifiers, naturally occurring genes that compensate for mutations responsible for severe diseases. Our robust technologies, reproducible approach and high-resolution insights into disease pathways enable us to develop small-molecule compounds that start with genetic modifiers to provide new therapeutic concepts. By understanding the genome's power to correct toward health, Scenic Biotech will bring novel therapeutics to patients in need.

For more information, please contact:

Scenic Biotech

Oscar Izeboud, PhD, CEO

Phone: +31 6 51 06 27 97

Email: oscar.izeboud@scenicbiotech.com

Trophic Communications

Eva Mulder / Desmond James

Phone: +31 6 52 33 15 79 or +49 151 6785 9086

Email: scenic@trophic.eu