



Scenic Biotech and the Barth Syndrome Foundation Announce Partnership to Explore Genetic Modifiers to a Find Tailored Treatment for the Complex Rare Disease

Amsterdam, The Netherlands, and Boston, MA, USA - 30 March 2022 - Scenic Biotech BV ('Scenic' or 'the Company'), a pioneer in the discovery of genetic modifiers to enable the development of disease modifying therapeutics for rare genetic disorders and other devastating illnesses, and the Barth Syndrome Foundation (BSF or 'the Foundation'), are proud to announce that they have entered into a partnership to support the advancement of Scenic's in-house drug discovery program to find novel tailored treatments for Barth syndrome, a devastating multi-system disorder that leads to complex clinical manifestations and significantly reduced life expectancy.

Leveraging its Cell-Seq discovery platform, Scenic has generated a pipeline of disease modifying therapeutics including three metabolic programs. These aim to identify small molecule drugs to treat Niemann Pick Type C (NP-C), a rare lipid storage disorder that affects lipid metabolism, a second undisclosed program to treat a severe heritable metabolic syndrome, and Barth syndrome program.

Barth syndrome is a rare, X-linked, inborn error of metabolism characterized by cardiolipin deficiency, cardiomyopathy, musculoskeletal weakness, neutropenia, debilitating fatigue, growth delay, and hypoglycemia, among other clinical manifestations. The disease most commonly affects males but has been reported in females and is associated with a genetic mutation in the TAFAZZIN gene causing abnormal cardiolipin remodeling and impaired mitochondrial structure.

With an incidence estimated to be 1 in 300,000 to 1 in 400,00 in the United States and affecting all ethnic groups, over 70% of Barth syndrome affected individuals present with cardiac complications, 17% are heart-transplant recipients, 69% further rely on immune modulation to manage neutropenia and infections, and most report constant and debilitating fatigue.¹

"Barth syndrome is a multi-faceted and life-threatening disease with no specific treatment, thus presenting immense unmet medical need in terms of healthcare management and access to therapies" says Erik Lontok, Ph.D., Director of Research for the Barth Syndrome Foundation. "In funding research and supporting stakeholders across the R&D spectrum, BSF remains an engaged partner in preclinical efforts and studies needed to understand the potentially outsized impact of genetic modifiers for Barth syndrome," he added.

In line with Scenic's focus on identifying genetic modifiers, also known as disease suppressors, these novel therapeutics work by suppressing or completely blocking the effect of a disease-causing mutated gene. This approach should result in the amelioration of symptoms across the complex clinical spectrum of Barth syndrome.

"Our approach to Barth syndrome aims to identify disease modifying treatments and is highly specific to the etiology of disease, with the goal of positively impacting the broad range of symptoms that affect individuals." says Sebastian Nijman Ph.D., co-founder and Chief Scientific Officer of Scenic Biotech.

Although the program now is in pre-clinical stages, Scenic and BSF are confident in the potential of genetic modifiers to one day play an important therapeutic role in Barth syndrome. In working together, both teams will seek to leverage the Foundation's precompetitive assets such as its TFAZZIN Human Variants Database and patient-inputted registry, while facilitating access to the established research models, biosamples, and the wealth of multi-disciplinary expertise within the Barth syndrome space.

"For our global community of 250+ affected individuals, advancements, and partnerships, like ours with Scenic, are critical to increasing knowledge and fostering hope for our ultra-rare disease indication. Our vision is a world in which Barth syndrome no longer causes suffering or loss of life," added Dr Lontok.

¹ [Voice of the Patient](#): Barth Syndrome. A report on the Externally-Led Patient-Focused Drug Development Meeting, March 2019.

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Notes to Editors:

About Barth Syndrome Foundation (BSF)

Barth Syndrome Foundation and our international affiliates comprise the only global network of families, healthcare providers, and researchers focused on our mission to save lives through education, advances in treatment and finding a cure for Barth syndrome. Considered a role model in rare disease advocacy, BSF has funded nearly \$6.1M USD to over 52 global institutions and catalyzed over \$32M USD in funding from other agencies to advance global scientific discoveries to end the suffering and loss of life from Barth syndrome. Additionally, BSF provides a lifeline to families and individuals living with Barth syndrome around the world by offering support, educational conferences, a patient registry and collaborations with specialist healthcare providers to advance rapid and proper diagnosis, standards of care, and treatments.

For further information, please visit: <https://www.barthsyndrome.org/>

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About Scenic Biotech

Scenic Biotech is focused on identifying genetic modifiers, a completely new class of disease targets, for drug intervention. Also known as disease suppressors, genetic modifiers are genes that act to suppress or completely block the effect of a disease-causing mutated gene.

As a pioneer in the field, Scenic Biotech is leveraging its Cell-Seq discovery platform to identify genetic modifiers across multiple therapeutic areas. It is building a pipeline of disease modifying therapeutics to treat devastating diseases including inherited rare diseases and cancer. Its lead programs include an immuno-oncology program targeting QPCTL, and three rare diseases programs based on druggable genetic modifiers. The rare disease programs consist of: Niemann Pick disease, type C (NP-C1), a rare lipid storage disorder that affects lipid metabolism, or the way fats, lipids, and cholesterol are transported in human cells, Barth syndrome, an X-linked, lipid metabolism disorder that affects the heart, muscle and immune cells, and a program to treat a severe heritable metabolic syndrome.

Scenic Biotech has a multi-year strategic collaboration with Genentech, a member of the Roche Group, to discover, develop and commercialize novel therapeutics that target genetic modifiers.

Headquartered in Amsterdam, The Netherlands, Scenic Biotech was founded in 2017 as a spin-out of the Netherlands Cancer Institute, and Oxford University. It is backed by a pan-European syndicate of Venture Capital investors that include Eir Ventures, BioMedPartners, Vesalius Biocapital, BioGeneration Ventures, Inkef Capital and Oxford Science Enterprises.

‘Scenic Biotech’ refers to all companies within the Scenic group, comprising Scenic Holding BV and its two fully owned subsidiary companies: Scenic Immunology BV and Scenic Biotech BV.

For further information, please visit: www.scenicbiotech.com

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