Scenic Biotech Publishes Novel Drug Target for Mitochondrial and Cardiac Disease in *Nature*

- **Second *Nature* publication in 3 months highlights strength of Scenic's proprietary Cell-Seq platform to identify modifier genes that positively influence disease severity**

Amsterdam, the Netherlands, September 03, 2025 – Scenic Biotech, a pioneer in the field of modifier therapies for severe diseases, today announced a publication in *Nature* on the role of ABHD18 as a missing link in the cardiolipin biosynthesis pathway. Cardiolipin is the signature phospholipid essential for mitochondrial function and implicated in both rare and common diseases. In a collaboration between Scenic and Dr. Jason Moffat's team at The Hospital for Sick Children (SickKids) in Toronto, the study aimed to identify modifier genes for Barth Syndrome (BTHS), an inborn error of cardiolipin metabolism, characterized by early onset cardiac and skeletal myopathy. Scenic's proprietary Cell-Seq platform, enabling the systematic discovery of genes that alter or buffer the consequences of disease-causing mutations, identified ABHD18 as a novel therapeutic target. Loss of ABHD18 was found to help overcome the severe disease characteristics caused by the BTHS-associated TAZ gene, offering new insights into potential therapeutic strategies. The study, titled "Genetic suppression features ABHD18 as a Barth Syndrome therapeutic target," is now available here.

"Our second peer-reviewed publication in *Nature* in a year highlights the robust and reproducible power of Scenic's Cell-Seq platform to uncover disease-modifying genes that are otherwise inaccessible with conventional approaches," said **Oscar Izeboud, CEO of Scenic Biotech**. "While we remain focused on advancing our lead candidate targeting PLA2G15, this research provides further validation of our platform's proven ability to discover modifier genes like ABHD18 that offer entirely new avenues for therapeutic intervention. By focusing on the biology that naturally counteracts disease, our platform identifies potential drug targets and reveals nature's blueprints for resilience."

"This study showcases the function of a modifier gene in a rare disease like Barth Syndrome. Rather than attempting to repair the faulty TAZ gene directly, we identified and targeted this secondary gene that offsets the effects of TAZ deficiency," stated **Dr. Jason Moffat, Program Head and Senior Scientist in the Genetics and Genome Biology Program at The Hospital for Sick Children (SickKids), and Professor in the Department of Molecular Genetics, University of Toronto**. "Deactivation of ABHD18 rescued mitochondrial defects in cells and achieved full suppression of all Barth Syndrome disease hallmarks in preclinical models. This approach effectively rewires mitochondrial function and offers a promising new therapeutic approach grounded in the biology of genetic suppression."

Dr. Vincent Blomen, Sr. Director Discovery Sciences at Scenic Biotech added: "We show that ABHD18 plays a crucial role in the remodeling and maturation of cardiolipin, positioning it as a key regulator of mitochondrial integrity and a compelling target for therapeutic intervention. In addition to rare mitochondrial disorders, impaired cardiolipin remodeling has been implicated in a broad range of conditions, including cardiac and kidney disorders, underscoring the potential relevance of this pathway in common diseases."

About Scenic Biotech
Scenic Biotech is advancing modifier therapy, a radical new approach to treating genetic disorders. Instead of targeting the primary disease-causing mutation, modifier therapy seeks to rebalance health by acting on another gene that can improve or, in some cases, even bypass the disease impact, leading to a therapeutic effect. Our robust pipeline, derived from our proprietary Cell-Seq platform, includes small molecule programs that are wholly owned. In addition, our platform is leveraged through strategic collaborations with multinational pharmaceutical leaders. By unlocking new pathways in the genome, Scenic Biotech is developing a range of modifier therapies to help patients.

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