Intergalactic Therapeutics Announces Positive Preclinical Results of Its Non-Viral Gene Therapy Platform in *ABCA4* Retinopathies

Data for IG-002 show for the first time that a single subretinal administration of a DNA payload encoding the human ABCA4 gene resulted in durable expression of human ABCA4 protein

Results support plan to advance lead program in ABCA4-related retinopathies into the clinic in 2024 and expansion into other ophthalmology indications

CAMBRIDGE, Mass., March 15, 2023 – Intergalactic Therapeutics, a company focused on transforming medicine through non-viral gene therapy, announced today positive preclinical results from its lead program IG-002 addressing all forms of *ABCA4*-related retinopathies. The data demonstrate for the first time that a single subretinal administration of a DNA payload encoding the human *ABCA4* gene resulted in durable (12-month) expression of human ABCA4 protein in adult porcine retinas. The expression levels achieved are believed to be sufficient to result in therapeutic benefit in individuals living with *ABCA4*-related retinopathies. Based on the encouraging results, the company plans to advance its IND-enabling studies and future clinical development.

In the study, full-length *ABCA4* DNA was packaged into Intergalactic's proprietary C^3DNA (covalently closed and circular DNA) cargo platform, and was delivered using COMET, Intergalactic's proprietary system for targeted delivery of C^3DNA. Full results from the study will be presented at an upcoming medical meeting.

"We are highly encouraged by the promising and unprecedented findings from these preclinical studies," said **José Lora, Ph.D.**, Chief Scientific Officer of Intergalactic Therapeutics. "With these data, we have clearly demonstrated the feasibility of electro-transfer delivery of non-viral C^3DNA expressing the full-length human *ABCA4* gene to relevant cell types in the retina. We have also confirmed persistence of *ABCA4* expression for at least 12 months *in vivo* in photoreceptors – the longest time point evaluated to date. These findings underscore the promise of Intergalactic's innovative non-viral approach to expanding the gene therapy universe and address the unmet needs of patients with *ABCA4*-related retinopathies and other historically untreatable diseases. We are eager to advance our research and move this promising program toward clinical development."

"As a retina specialist with expertise in inherited retinal degeneration as well as a number of acquired eye conditions, I believe non-viral gene therapy offers significant potential in this difficult to treat area," said **Mark Pennesi, M.D., Ph.D.**, Professor of Ophthalmology and Chief at OHSU Casey Eye Institute and a member of Intergalactic's Ophthalmology Advisory Panel. "These findings provide proof of concept and further hope that non-viral gene therapy may revolutionize the field for patients who currently have limited or no treatment options."

A Unique Combination of Proprietary Technologies

Intergalactic's non-viral gene therapy platform is designed to overcome limitations of standard adeno-associated virus (AAV) gene therapy technologies, including vector capacity limitations and vector-related safety concerns, and unlock the potential of non-viral gene therapies for a wide range of diseases. Specifically, C^3DNA is designed to enable therapeutic delivery of large genes, while eliminating safety concerns associated with viral vectors and allowing the potential for redosing. COMET is a precise and tunable electro-transfer-based method for local delivery of C^3DNA payloads to various tissues of interest.

Due to high unmet need, amenability to local gene delivery, and the significant limitations of viral-based gene therapy, Intergalactic has prioritized ophthalmology and is advancing a lead program targeting *ABCA4*-related retinopathies. These retinopathies, the most common inherited retinal disease, currently have no approved treatments.

Intergalactic plans to move its lead *ABCA4* program into the clinic in 2024. The company also has pipeline programs targeting other ophthalmology indications and is evaluating the broader applicability of the platform to other tissue types.

"Intergalactic's mission is to overcome the limitations of viral-based gene therapy and develop a best-in-class non-viral alternative," said **Theresa G.H. Heah, M.D., M.B.A.**, Chief Executive Officer of Intergalactic Therapeutics. "We look forward to continuing to advance our IND-enabling *ABCA4* studies into the clinic and progressing additional pipeline programs where our innovative approach holds the potential to unlock the promise of non-viral gene therapy for patients."

About *ABCA4*-Related Retinopathies

ABCA4-related retinopathies, including Stargardt disease, cone-rod dystrophy, and retinitis pigmentosa, lead to degeneration of rods and cones and cause vision loss that often starts in childhood or adolescence, ultimately leading to blindness. These diseases are associated with mutations in the *ABCA4* gene, more than 900 of which have been described. There are currently no approved treatments for *ABCA4*-related retinopathies and, while replacement with wild-type ABCA4 protein has the potential to slow or stop disease progression, standard AAV gene therapy platforms are not appropriate for addressing these diseases due to the large size of the gene and the growing safety concerns associated with viral vectors. Intergalactic's novel platform bypasses these drawbacks and enables local delivery of non-viral gene therapy, representing a potentially transformative approach for addressing these blinding diseases.

About Intergalactic Therapeutics

Intergalactic Therapeutics, an Apple Tree Partners (ATP) company, is transforming medicine through non-viral gene therapy. Its proprietary integrated platform comprises three core pillars: versatile C^3DNA technology (covalently closed and circular DNA); groundbreaking precise and tunable approaches to local therapeutic delivery using the COMET electro-transfer system; and a rapid, scalable, and cost-effective manufacturing process to make gene therapy safer and more accessible. With a diversified portfolio of potential new treatments for eye diseases and beyond, Intergalactic is dedicated to helping patients around the world by bringing non-viral gene therapies into reality. For more information, visit www.intergalactictx.com.

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