



## **Intergalactic Therapeutics Announces Positive Results for Its Non-Viral Gene Therapy Platform in *ABCA4* Retinopathies in Non-Human Primates**

***Data from IG-002, Intergalactic's lead program, demonstrate persistent *ABCA4* expression in non-human primate retinas over six months after a single administration***

***Findings build on 12-month durability data achieved in adult porcine retinas also after a single administration***

***Data support plan to advance IG-002 into Phase 1 trials in 2024***

**CAMBRIDGE, Mass., June 27, 2023** – [Intergalactic Therapeutics](#), a company focused on transforming medicine through non-viral gene therapy, today announced positive preclinical data from IG-002, its lead program addressing all forms of *ABCA4*-related retinopathies. 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.

- In the non-human primate study, full-length human *ABCA4* was encoded into Intergalactic's proprietary C<sup>3</sup>DNA (covalently closed and circular DNA) vector platform and was delivered using COMET, Intergalactic's proprietary system for electro-transfer based, non-viral delivery of DNA.
- The new data represent the first time that a single subretinal administration of a DNA payload encoding the human *ABCA4* gene has been shown to safely achieve durable 6-month expression of human *ABCA4* in non-human primate retinas. These data follow results from a previous Intergalactic study demonstrating 12-month durability in adult porcine retinas. IG-002 is currently in IND-enabling studies, with plans to advance into Phase 1 trials in 2024 as the company simultaneously expands into other ophthalmology indications.
- Full results from this study will be presented at an upcoming scientific meeting.

“These latest results reinforce previous findings from our porcine study but now show durable expression in non-human primates, whose maculae most closely resemble the human eye,” said **José Lora, Ph.D.**, Chief Scientific Officer of Intergalactic Therapeutics. “We are highly encouraged by these findings, which contribute to the growing body of evidence supporting the safety and durability of our non-viral gene therapy platform as we advance toward the clinic. The new data show again how our non-viral, C<sup>3</sup>DNA + COMET platform facilitates electro-transfer based delivery of substantial genetic cargo to relevant cell types in the retina with persistent expression and using a single subretinal administration.”

“Intergalactic’s novel platform represents a potentially transformative approach for addressing blinding ophthalmological disorders and other serious diseases,” said **Theresa G.H. Heah, M.D., M.B.A.**, Chief Executive Officer of Intergalactic Therapeutics. “We look forward to advancing IG-002 into the clinic in 2024 to unlock the promise of non-viral gene therapy for patients with few or no therapeutic 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, to deliver non-viral gene therapies for a wide range of diseases. C<sup>3</sup>DNA 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 platform for local delivery of C<sup>3</sup>DNA payloads to various tissues of interest.

Ophthalmological disease is Intergalactic’s priority area of focus given high unmet patient needs, the eye’s amenability to local gene delivery, and the significant limitations of viral-based gene therapy approaches. The company is advancing a lead program targeting *ABCA4*-related retinopathies. These retinopathies, the most common inherited retinal diseases, currently have no approved treatments.

## **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 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<sup>3</sup>DNA 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 <https://intergalactictx.com/>.

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