

MEDIA UPDATE

Novartis, Voyager Therapeutics reach license option agreement for next-generation gene therapy vectors for neurological diseases

- *Novartis gains target-specific access to next-generation AAV capsids that could be used for three diseases of the central nervous system, with options for two additional targets*
- *Agreement builds on previous Novartis success using emerging technologies to develop first-in-class gene therapies for neurological disorders*
- *Capsids from Voyager's TRACER platform may help deliver therapies to previously intractable regions of the brain, potentially overcoming barriers that have hindered development of gene therapies for neurological diseases to date*

Basel, March 8, 2022 — Novartis today announced a license option agreement with Voyager Therapeutics, a gene therapy company focused on next-generation adeno-associated virus (AAV) technologies, for three capsids to use in potential gene therapies for neurological diseases, with options to access capsids for two other targets.

A leader in gene therapy with a strong commitment to developing treatments for diseases of the central nervous system, Novartis seeks to apply its expertise and recent learnings to neurological diseases that manifest in deep brain regions where commonly used vectors have not been shown to effectively target and penetrate.

“The challenge of biodistribution of AAV gene therapies to target cell types in the brain is longstanding, but we refuse to believe it is insurmountable,” said Jay Bradner, President of the Novartis Institutes for BioMedical Research (NIBR). “We see the potential of Voyager’s innovative platform to imagine new types of therapeutics for life-threatening CNS diseases.”

Data suggest that AAV capsids emerging from Voyager’s proprietary TRACER platform (which stands for Tropism Redirection of AAV by Cell type-specific Expression of RNA) may show broad and improved distribution to neurons in the cortex and deeper brain regions.

“We have a robust pipeline of gene therapy programs to target diseases of the central nervous system – a number of them requiring enhanced delivery vectors that reach cells in multiple brain regions, which has not been possible with technologies used to date,” said Bob Baloh, Head of Neuroscience at NIBR. “We look forward to evaluating these capsids from Voyager’s TRACER platform, which hopefully will enable us to bring new therapeutic options to patients with devastating neurological conditions.”

Under the terms of the agreement, Novartis will have the right to evaluate novel capsids from Voyager's TRACER platform and to exercise options to license capsids for exclusive use in Novartis' development of AAV gene therapies directed to three targets. Novartis has the right to license capsids for two additional disease targets under the same terms. Voyager will receive an upfront payment, with additional payments for each exercised option, as well as future milestones and sales-based royalties.

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