Voyager Therapeutics Announces License Option Agreement with Novartis for Target-Specific Access to Next-Generation TRACER™ AAV Capsids for Gene Therapy Programs

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Voyager receives $54 million upfront with potential option exercise fees and milestone payments of up to $1.7 billion plus product sales-based royalties

Novartis receives target-specific access to Voyager's novel TRACER AAV capsids for potential use with three CNS targets plus options to access capsids for two additional targets

Agreement marks the second recent major transaction leveraging Voyager's TRACER capsid discovery platform

Conference call and webcast today at 8:30 a.m. ET

CAMBRIDGE, Mass., March 08, 2022 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a gene therapy company developing life-changing treatments and next-generation adeno-associated virus (AAV) capsids, today announced an agreement through which Novartis (NYSE: NVS) may exercise options to license novel AAV capsids generated from Voyager's RNA-driven TRACER ™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) capsid discovery platform for potential use with three undisclosed CNS targets and options to access capsids for two additional targets to be agreed on in the future.

Voyager will receive $54 million upfront and is entitled to receive up to $37.5 million in exercise fees for options for three initial CNS targets, exercisable by Novartis within 12 months of signing. In addition, Novartis may elect to evaluate capsids for up to two additional targets to be agreed on in the future, subject to their availability, for $18 million upon selection of each target, and a $12.5 million exercise fee for selection of a capsid for each target. Voyager is also eligible to earn up to $1.5 billion in potential development, regulatory, and commercial milestones for products utilizing Voyager licensed capsids, as well as mid- to high-single-digit tiered royalties based on net sales of Novartis products incorporating the licensed capsids.

"This transaction further validates the potential of the Voyager TRACER capsid discovery platform to broadly enable a next generation of AAV gene therapies," said Allen Nunnally, chief business officer of Voyager. "Our collaboration with Novartis expands the array of therapeutic programs in which our proprietary capsids may be deployed and highlights the potential of our TRACER platform to generate future business development opportunities as our novel capsid library expands and initial TRACER-derived capsids are further refined to enhance desirable characteristics."

"Overcoming the substantial toxicity risk frequently observed with high doses of AAV-based gene therapies remains a critical challenge to realizing the full, curative potential of this breakthrough modality," said Glenn Pierce, M.D., Ph.D., interim chief scientific officer of Voyager. "Voyager's growing and maturing library of proprietary TRACER-derived capsids have demonstrated markedly enhanced expression in non-human primates with more precise targeting of desired tissue and cell types, creating the potential for superior delivery and fewer off-target risks than conventional AAV."

Under the terms of the agreement, Novartis has the right to evaluate novel capsids from Voyager's TRACER platform and to exercise options to license capsids for exclusive use with specific targets in Novartis' development of AAV gene therapies. The targets for which Novartis receives rights under the agreement are distinct from those in Voyager's internal and partnered pipeline, and Voyager retains global rights to its TRACER discovery platform as well as all capsids arising from it for use with other targets, subject to Voyager's obligations under its agreement with Pfizer announced in October 2021 for access to TRACER capsids for use with one neurologic and one cardiovascular target.

An initial set of proprietary AAV capsids derived from Voyager's TRACER platform have demonstrated superior blood-brain barrier penetration, increased transduction in the brain and spinal cord, enhanced cardiac muscle tropism, and increased transgene expression in target tissues compared to conventional AAV capsids when dosed intravenously in non-human primates (NHPs).

- A capsid from this initial set demonstrated more than 1,000-fold increased transgene expression levels (as measured by mRNA level) compared to conventional AAV9 across a wide array of brain regions when dosed intravenously in NHPs.
- A separate capsid showed significantly enhanced cardiac muscle transduction and dorsal root ganglia detargeting compared to conventional AAV9 when dosed intravenously in NHPs.

Subsequent TRACER screening campaigns have identified a new class of AAV9 variants selective for glial cells, which may enable more precise targeting of CNS diseases affecting non-neuronal cells, as well as AAV5 capsid variants with enhanced brain and spinal cord tropism, compared to conventional AAV9 and AAV5. Voyager is performing further screening with its TRACER capsid discovery platform to identify additional proprietary AAV9- and AAV5-derived capsids targeting multiple tissue and cell types, as well as further refining identified capsids to enhance desirable characteristics for use in gene therapies to treat a broad range of diseases.

Conference Call
The Voyager Therapeutics leadership team will host a conference call and webcast today at 8:30 a.m. ET to discuss the Novartis license option agreement, and provide and discuss fourth quarter and full year 2021 financial and operating results. To access the call, please dial (877) 851-3834 (domestic) or (631) 291-4595 (international) and ask for the Voyager Therapeutics earnings call. A live webcast of the call will also be available on the Investors section of the Voyager website at ir.voyagertherapeutics.com, and a replay will be available at the same link approximately two hours after its completion. The replay will be available for at least 30 days following the conclusion of the call.

About the TRACER™ AAV Capsid Discovery Platform
Voyager's TRACER ™ capsid discovery platform is a broadly applicable, RNA-based functional screening platform that allows for rapid in vivo evolution of AAV9- and AAV5-derived capsids with enhanced tropisms and cell- and tissue-specific transduction properties in multiple species, including non-human primates (NHPs). Initial data from the first of many libraries screened in NHPs demonstrated the proprietary capsid variants

effectively penetrated the blood-brain barrier and achieved widespread biodistribution and transduction of multiple regions of the brain. Separate results have demonstrated the ability of certain capsids to transduce cardiac muscle and to de-target the dorsal root ganglia. Voyager is proceeding with additional capsid campaigns derived from AAV9, AAV5, and other capsid serotypes to identify novel AAV vectors optimized for specific therapeutic applications.

About Voyager Therapeutics
Voyager Therapeutics (Nasdaq: VYGR) is leading the next generation of AAV gene therapy to unlock the potential of the modality to treat devastating diseases. Proprietary capsids born from the Company's TRACER discovery platform are powering a rich early-stage pipeline of new and second-generation programs and may elevate the field to overcome the narrow therapeutic window associated with conventional gene therapy vectors across neurologic disorders and other therapeutic areas. voyagertherapeutics.com LinkedIn Twitter

Forward-Looking Statements
This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as "may," "might," "will," "would," "should," "expect," "plan," "anticipate," "believe," "estimate," "undoubtedly," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify forward-looking statements.

For example, all statements Voyager makes regarding Voyager's ability to continue to identify and develop proprietary capsids from its TRACER capsid discovery platform with increased transgene expression, increased blood-brain barrier penetration and increased biodistribution compared to conventional AAV9 capsids; Voyager's ability to utilize its proprietary capsids in its own product development programs; Voyager's ability to attract parties to license its novel proprietary capsids or to participate with Voyager in research and development collaborations utilizing its novel proprietary capsid; Voyager's ability to advance its AAV-based gene therapy programs; Voyager's entitlement to receive upfront, option exercise, milestone and royalty-based fees from Novartis under the license option agreement; and Voyager's ability to enter into new partnerships or collaborations; and Voyager's sufficient cash resources to enable it to continue to identify and develop proprietary capsids from its TRACER capsid discovery platform are forward-looking.

All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager believes such forward-looking statements to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Voyager expected. Such risks and uncertainties include, among others, the continued development of various technology platforms, including Voyager's TRACER capsid discovery platform; the development by third parties of capsid identification platforms that may be competitive to Voyager's TRACER capsid discovery platform; Voyager's scientific approach and general development progress; Voyager's ability to attract and retain talented contractors and employees to continue the development of the TRACER capsid discovery platform and the identification of proprietary capsids; Voyager's ability to create and protect intellectual property rights associated with the TRACER capsid discovery platform and the capsids identified by the platform; the sufficiency of cash resources; the possibility or the timing of the exercise of development, commercialization, license and other options under the Novartis license option agreement and other collaborations; the ability of Voyager to negotiate and complete licensing or collaboration agreements on terms acceptable to Voyager and third parties; Voyager's ability to perform its obligations under its license option agreement with Novartis and Novartis's ability to perform its obligations under such agreement; Voyager's ability to generate sufficient cash resources to enable it to fund its operations and development plans; and the availability or commercial potential of Voyager's product candidates. These statements are also subject to a number of material risks and uncertainties that are described in Voyager's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, as updated by its subsequent filings with the Securities and Exchange Commission. All information in the press release is as of the date of this press release, and any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise this information or any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

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