



Voyager Therapeutics Transforms Pipeline and Increases Investment in Next-Generation TRACER™ AAV Capsid and Vectorized Antibody Platform Technologies

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Proprietary AAV capsids to power second-generation efforts in Huntington's disease and ALS, pre-clinical programs in spinal muscular atrophy and diseases linked to GBA1 mutations

Increased platform investment to expand discovery of novel targeted AAV capsids and clinical application of vectorized antibodies

Company reports second quarter 2021 financial results; extends cash runway into early 2023

CAMBRIDGE, Mass., Aug. 09, 2021 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (Nasdaq: VYGR), a gene therapy company developing life-changing treatments and next-generation platform technologies, today introduced new programs in Huntington's disease, a monogenic form of ALS (SOD1), spinal muscular atrophy, and diseases linked to GBA1 mutations, all powered by its proprietary AAV capsids that have demonstrated superior transgene expression in the brain compared to AAV9 delivery in non-human primates. The Company also announced increased investment in its RNA-driven TRACER (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) AAV screening technology to expand discovery of novel capsids with broad tissue tropism in CNS, cardiac, and skeletal tissues.

"Given recent breakthroughs with our platform technologies, Voyager has transformed its pipeline to focus on advancing innovative gene therapies that leverage our proprietary next-generation AAV capsids and vectorized antibodies. In parallel, we are bolstering investment in our TRACER platform to produce additional targeted capsids with enhanced tissue- and cellular-specificity and to expand the potential of AAV-mediated genetic delivery of antibody therapies," said Michael Higgins, Interim CEO of Voyager. "Voyager's capsids have achieved superior transduction in targeted tissues over AAV9 in non-human primates, and we strongly believe that our platform has opened up a wide array of opportunities in CNS, as well as other tissues. Because our novel capsids have significant potential to be more reliably on-target with less risk of dose-limiting toxicities, we believe they could enable a new generation of gene therapies developed internally, as well as by collaborators or licensees."

"After a rigorous and thoughtful evaluation of existing programs, we have refocused the Company on delivery technologies to power new and second-generation programs which we believe have robust target validation, an efficient path to preclinical and clinical proof of concept, and may provide meaningful therapeutic benefit," said Glenn Pierce, M.D., Ph.D., Interim CSO of Voyager. "As part of the evaluation, we have discontinued our first-generation Huntington's disease program and initiated a second-generation program using a novel, proprietary AAV capsid that may enable intravenous administration and achieve widespread distribution to affected tissue. We firmly believe that HD remains an ideal target and that applying our clinical development experience to introduce a new approach creates the opportunity to confer greater patient benefit with a more favorable safety profile. We are grateful to the HD community for supporting our research and remain fully committed to advancing our work to serve patients and those who care for them."

Pipeline and Platform Updates

Pipeline Re-evaluation Yields High-Potential Programs

- Voyager believes its proprietary AAV capsids may enable new, best-in-class programs for spinal muscular atrophy and diseases linked to GBA1 mutations given initial data demonstrating its capsids are more reliably on-target with less risk of dose-limiting toxicities than existing AAV capsids. Second-generation and new pre-clinical programs have been initiated in the following areas:
 - Huntington's disease (second generation)
 - Monogenic ALS (SOD1) (second generation)
 - Spinal muscular atrophy
 - Diseases linked to GBA1 mutations
- Internal development of first-generation, surgically-based gene therapy programs for the treatment of Huntington's disease and monogenic ALS (SOD1) have been discontinued with efforts being redirected to second-generation programs.
- The Company is exploring various indications across the spectrum of GBA1 mutations, including Parkinson's disease, Lewy body dementia, and Gaucher disease, as well as vectorized antibodies to explore undisclosed indications in neuro-oncology.
- Voyager's current program in Friedreich's ataxia remains partnered with Neurocrine Biosciences, Inc., and the Company's wholly-owned vectorized antibody program focused on various tauopathies continues as planned.

TRACER AAV Capsid Discovery Platform Demonstrates Superior CNS Targeting

- The TRACER platform is designed to identify novel capsids that overcome the limitations of existing AAV capsids. The platform may be flexibly applied to discover capsids with enhanced tropism to target tissues, such as the CNS, and cardiac and skeletal muscle that may lead to treatments for a broad range of diseases.
- Initial TRACER capsid data have demonstrated robust delivery across the blood-brain barrier and widespread CNS transduction in non-human primates compared to AAV9 delivery, following intravenous (IV) administration. Capsid 9P801

displayed more than 1,000-fold higher transgene expression in the brain compared to AAV9 delivery in non-human primates.

- Capsid 9P804 displayed strong cardiac transduction and significant dorsal root ganglia detargeting, which may avoid toxicities associated with AAV9 delivery.
- Voyager is proceeding with screens of nine additional AAV capsid campaigns to further expand its capsid library and identify capsids optimized for specific applications.

Vectorized Anti-Tau Antibody Platform Demonstrates Durable CNS Expression

- Initial preclinical data have demonstrated a reduction of pathological tau with Voyager's vectorized anti-tau antibody and durable expression in CNS.
- The Company's vectorized antibodies may represent a new single-dose therapeutic strategy for treating various tauopathies, including progressive supranuclear palsy and frontotemporal dementia.
- Voyager has developed modular antibody vectorization cassettes, which consist of an AAV vector and a transgene encoding anti-tau monoclonal full-length antibodies. Following IV administration and transduction of target cells in the brain, the expressed antibodies are designed to be functionally reconstituted and subsequently secreted into the parenchyma, thereby enabling potential therapeutic benefit for multiple CNS diseases.

Corporate Updates and Anticipated Milestones

Leadership Transitions

- In May, the Company announced the appointment of Michael Higgins as interim chief executive officer and Glenn Pierce, M.D., Ph.D., as interim chief scientific officer. Mr. Higgins has served on Voyager's board of directors since July 2015 and has been chair of the board since June 2019. He is also the chair of the board of Pulmatrix and a board member for Genocea Biosciences, Nocion Therapeutics, Camp4 Therapeutics, and Sea Pharmaceuticals. Dr. Pierce has been a member of the board of directors since January 2017. He serves as entrepreneur-in-residence at Third Rock Ventures, where he co-founded Ambys Medicines. Dr. Pierce previously served in a number of roles at Biogen, most recently as chief medical officer leading the hematology, cell, and gene therapies division. He is the co-author of more than 150 scientific papers and has received more than 15 patents.

Upcoming Events and Presentations

- Voyager plans to participate in the following investor events:
 - Wedbush Pacgrow Healthcare Conference, August 11, 2021
 - Canaccord Genuity Growth Conference, August 12, 2021
 - Wells Fargo Virtual Healthcare Conference, September 9, 2021
 - Morgan Stanley Global Healthcare Conference, September 14, 2021
 - Baird Healthcare Conference, September 15, 2021

Second Quarter 2021 Financial Results

- **Collaboration Revenues:** Collaboration revenue was \$1.4 million for the second quarter of 2021, compared to collaboration revenue of \$28.7 million for the same period of 2020. The decrease in collaboration revenue was largely due to a reduction of revenue related to research services and cost reimbursements from the collaborations with Neurocrine and AbbVie. In February 2021, Neurocrine provided notice that, effective August 2, 2021, it was terminating its participation in the VY-AADC program for Parkinson's disease under the collaboration agreement between the Company and Neurocrine, and that wind-down activities, including the termination by Neurocrine of its support for on-going development activities for the VY-AADC program would commence immediately. Additionally, the collaborations with AbbVie were terminated in August 2020.
- **Net Loss:** Net loss was \$30.1 million for the second quarter of 2021, compared to a net loss of \$8.7 million for the same period of 2020.
- **R&D Expenses:** Research and development expenses were \$19.5 million for the second quarter of 2021, compared to \$29.4 million for the same period in 2020. The decrease in R&D expenses was primarily related to lower manufacturing and clinical expenses for the VY-AADC program for Parkinson's disease.
- **G&A Expenses:** General and administrative expenses were \$10.4 million for the second quarter of 2021, compared to \$8.2 million for the same period in 2020. The increase in G&A expenses was primarily related to the one-time recognition of severance and stock-based compensation expense related to a former key executive, and increased facility costs.
- **Cash Position:** Cash, cash equivalents and marketable debt securities as of June 30, 2021 were \$143.0 million.

Financial Guidance

- Based on the Company's current operating plan and excluding any potential financing or business development activities in

2021, Voyager anticipates cash, cash equivalents, and marketable debt securities will be between \$85 million and \$90 million at the end of 2021.

- Due in part to cost savings realized as a result of the pipeline re-evaluation, Voyager expects that its cash, cash equivalents and marketable debt securities will be sufficient to meet the Company's planned operating expenses and capital expenditure requirements into early 2023.

About the TRACER™ AAV Capsid Discovery Platform

Voyager's TRACER™ system is a broadly-applicable, RNA-based functional screening platform that allows for rapid *in vivo* evolution of AAV capsids delivered intravenously with cell-specific transduction properties in non-human primates (NHP). 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. Voyager is proceeding with screens of additional NHP campaigns from AAV9 and other capsid serotypes to enable the identification of novel AAV vectors optimized for specific therapeutic applications. Initial results have demonstrated the ability of certain capsids to transduce cardiac tissue and to detarget the dorsal root ganglia.

About Voyager Therapeutics

Voyager Therapeutics (Nasdaq: VYGR) is leading the next generation of AAV gene therapy to unlock the potential of the technology to treat devastating diseases. Proprietary capsids born from the Company's TRACER™ screening platform are powering a rich early-stage pipeline of new and second-generation programs and may elevate the field to overcome the limitations of conventional gene therapy vectors across neurological disorders and other therapeutic areas.

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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 the initiation, timing, progress, activities, goals and reporting of results of its research activities, preclinical and other development programs and clinical trials; the timing of planned presentations at medical, scientific or other conferences; its ability to identify and attract parties to participate in research and development collaborations; its ability to advance its AAV-based gene therapies into, and successfully initiate, enroll and complete, clinical trials; the potential clinical utility of its product candidates; the continuing progress in Voyager's development of novel AAV capsids; its ability to continue to develop its TRACER AAV capsid discovery platform; its ability to perform under existing collaborations including the potential benefits, timing and future operation of its collaboration with Neurocrine Biosciences; its ability to add new programs to its pipeline; the regulatory pathway of, and the timing or likelihood of its regulatory filings and approvals for, any of the Company's product candidates; its ability to operate its research and development activities efficiently and effectively; the utility and value of Voyager's patent portfolio; Voyager's anticipated financial results, including Voyager's available cash, cash equivalents and marketable debt securities and the receipt by Voyager of revenues or reimbursement payments from collaboration partners, Voyager's operating expenses; and Voyager's ability to fund its operating expenses with its current cash, cash equivalents and marketable debt securities though a stated time period 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 severity and length of the COVID-19 health crisis, the imposition of governmental controls and guidance addressing the COVID health crisis, and the financial and human resources available to Voyager to manage the COVID-19 health crisis; the initiation and conduct of research activities, preclinical studies and clinical trials; the availability of data from research activities, preclinical studies and clinical trials, and the ability to effectively present such data; the expectations for regulatory communications, submissions and approvals; the continued development of various technology platforms, including Voyager's TRACER platform; Voyager's scientific approach and general development progress; the ability to attract and retain talented contractors and employees; the ability to create and protect intellectual property; the sufficiency of cash resources; the possibility or the timing of the exercise of development, commercialization, license and other options under collaborations; the ability of Voyager to negotiate and complete licensing or collaboration agreements on terms acceptable to Voyager and third parties; 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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Selected Financial Information

(\$-amounts in thousands, except per share data)

(Unaudited)

Statement of Operations Items:	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2021	2020	2021	2020

Collaboration revenue	\$	1,357	\$	28,681	\$	7,858	\$	46,748
Operating expenses:								
Research and development		19,505		29,423		41,851		61,718
General and administrative		10,437		8,239		20,181		18,444
Total operating expenses		<u>29,942</u>		<u>37,662</u>		<u>62,032</u>		<u>80,162</u>
Operating loss		(28,585)		(8,981)		(54,174)		(33,414)
Total other (expense) income		<u>(1,535)</u>		<u>300</u>		<u>2,405</u>		<u>470</u>
Net loss	\$	(30,120)	\$	(8,681)	\$	(51,769)	\$	(32,944)
Net loss per share, basic and diluted	\$	(0.80)	\$	(0.23)	\$	(1.38)	\$	(0.89)
Weighted-average common shares outstanding, basic and diluted		37,581,381		37,029,524		37,543,387		36,996,390

Selected Balance Sheet Items	June 30,		December 31,	
	2021		2020	
Cash, cash equivalents, and marketable debt securities	\$	142,981	\$	174,782
Total assets	\$	208,727	\$	261,584
Accounts payable and accrued expenses	\$	11,749	\$	14,839
Deferred revenue	\$	39,725	\$	43,817
Total stockholders' equity	\$	110,536	\$	154,320



Source: Voyager Therapeutics, Inc.