



Codiak Presents New Preclinical Data at ASGCT 2022 on Exosome-Based Strategy to Enhance AAV Gene Therapy

May 17, 2022

Engineering exosomes to encapsulate AAV results in increased yield, resists antibody neutralization *in vitro* and retains transduction ability

CAMBRIDGE, Mass., May 17, 2022 (GLOBE NEWSWIRE) -- Codiak BioSciences, Inc. (Nasdaq: CDAK), a clinical-stage biopharmaceutical company pioneering the development of exosome-based therapeutics as a new class of medicines, today announced new preclinical data on the Company's engEx-AAV™ discovery program, a novel strategy that aims to leverage exosomes to improve adeno-associated virus (AAV) vector gene therapy. The data, which are being presented today at the 25th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), demonstrate that exosome engineering generates significant increases in AAV yield compared to unmodified exosomes, while retaining the functionality to transduce cells with AAV and resist neutralizing antibodies that impair gene therapy efficacy.

"These data show the power of our engineering platform to enable exosome delivery of AAV as a therapeutic and the potential of this approach to address key limitations associated with AAV gene therapy currently," said Sriram Sathyanarayanan, Ph.D., Chief Scientific Officer, Codiak. "We have identified the engineering methods and multiple exosome constructs that effectively 'catch' AAV capsids and 'release' them to cells, resulting in highly efficient transduction *in vitro*. By increasing the AAV yield with engEx-AAV, we have successfully cleared a significant hurdle, and as we advance this program, we will employ a variety of *in vivo* models to examine regional, systemic and repeat dosing."

AAV is a commonly used gene therapy vector whose "once and done" clinical potential faces challenges including the presence or subsequent development of neutralizing antibodies (nAbs) preventing treatment or re-dosing, waning transgene expression, and difficulties in producing sufficient quantities of AAV. Encapsulation of AAV within exosomes (engEx-AAV) represents a strategy to protect AAV from antibody-mediated neutralization and efficiently deliver AAV to specific cell types, leading to higher potency and lower systemic exposure. In an *in vivo* retinal model, engEx-AAV transduced cell types that were not affected in AAV-injected eyes, indicating engineered engEx-AAV exhibits a preferential tropism that could offer important utility in ocular disease indications.

The data presented at ASGCT describe the identification and characterization of engEx-AAV constructs engineered to increase AAV yield while retaining desired functionality. A proprietary peptide derived from the protein BASP1 was used as a scaffold to facilitate AAV loading into the lumen of the exosome through the use of a camelid nanobody (VHH) that binds AAV capsids. BASP1-VHH fusions improved exosome AAV capsid loading by 2-3 log orders, while retaining transduction activity. The highest performing engineered engEx-AAV constructs showed significant transduction compared to un-engineered controls.

A poster entitled "Generation of Potent and High-Yield Exosome-Associated AAV (exoAAV) Using Engineered Exosomes" (TU-119) will be presented today in the Exhibit Hall beginning at 5:30 p.m. ET and is available on the [Codiak website](#).

About the engEx™ Platform

Codiak's proprietary engEx Platform is designed to enable the development of engineered exosome therapeutics for a wide spectrum of diseases and to manufacture them reproducibly and at scale to pharmaceutical standards. By leveraging the inherent biology, function and tolerability profile of exosomes, Codiak is developing engEx exosomes designed to carry and protect potent drug molecules, provide selective delivery and elicit the desired pharmacology at the desired tissue and cellular sites. Through its engEx Platform, Codiak seeks to direct tropism and distribution by engineering exosomes to carry on their surface-specific targeting drug moieties, such as proteins, antibodies/fragments, and peptides, individually or in combination. Codiak scientists have identified two exosomal proteins that serve as surface and luminal scaffolds. By engineering the exosome surface or lumen and optimizing the route of administration, Codiak aims to deliver engEx exosomes to the desired cell and tissue to more selectively engage the drug target, potentially enhancing the therapeutic index by improving potency and reducing toxicity.

About Codiak BioSciences

Codiak is a clinical-stage biopharmaceutical company pioneering the development of exosome-based therapeutics, a new class of medicines with the potential to transform the treatment of a wide spectrum of diseases with high unmet medical need. By leveraging the biology of exosomes as natural intercellular transfer mechanisms, Codiak has developed its proprietary engEx Platform to expand upon the innate properties of exosomes to design, engineer and manufacture novel exosome therapeutic candidates. Codiak has utilized its engEx Platform to generate a deep pipeline of engineered exosomes aimed at treating a broad range of disease areas, spanning oncology, neuro-oncology, infectious disease and rare disease. For more information, please visit www.codiakbio.com.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, among other things, statements concerning the development and therapeutic potential of engEx-AAV. Any forward-looking statements in this press release are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. For a discussion of these risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in Codiak's Annual Report on Form 10-K for the year ended December 31, 2021, and in subsequent filings with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties and other important factors in Codiak's subsequent filings with the Securities and Exchange Commission. All information in this press release is current as of the date of this report, and Codiak undertakes no duty to update this information unless required by law.

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