Sarepta Therapeutics and Codiak BioSciences Collaborate to Research and Develop Exosome-Based Therapeutics for Rare Diseases

6/22/20

- Alliance to explore the utility of engineered exosomes developed with Codiak's engEx™ Platform to deliver gene therapy, gene editing and RNA technologies -

- Two-year, global research and option agreement covers up to five neuromuscular targets -

- Codiak is eligible to receive up to $72.5 million in upfront and near-term license payments plus research funding -

CAMBRIDGE, Mass., June 22, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, and Codiak BioSciences, Inc., a company at the forefront of advancing engineered exosomes as a new class of biologic medicines, today announced a global research and option agreement to design and develop engineered exosome therapeutics to deliver gene therapy, gene editing and RNA technologies for neuromuscular diseases. The engineered exosome approach offers the potential to effectively deliver genetic therapeutics without triggering the adaptive immune response.

The two-year agreement includes up to five neuromuscular targets. Codiak is eligible to receive up to $72.5 million in upfront and near-term license payments plus research funding. Sarepta is granted an option to any of the candidates developed pursuant to the research alliance.

Exosomes are natural nanoparticles that serve as the body's intercellular communication system, facilitating the transfer of a wide variety molecular payloads between cells. As they are derived from human cells, exosomes provide a unique advantage as a targeted delivery system for genetic medicines because they are inherently non-immunogenic. Through its proprietary, engEx Platform, Codiak can engineer exosomes with specific cargos and guide tropism to cell types of interest. The collaboration will leverage Codiak's exosome engineering capabilities with Sarepta's expertise in precision genetic medicine to develop next generation therapeutics for patients with neuromuscular diseases that have few or no treatment options.

"As Sarepta expands its leadership position in precision genetic medicine, this alliance with Codiak furthers our goal to deliver the most advanced therapies to patients. Codiak's engEx technology could potentially address some of the limitations of current treatment approaches and offers broad utility across Sarepta's therapeutic modalities -- gene therapies, gene editing and RNA. Codiak's exosomes are engineered for precise tissue targeting and offer a non-viral delivery approach with non-immunogenic potential, thus opening up avenues for more efficient delivery and potential re-dosing," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics.

"The development of targeted delivery systems that enable repeat-dosing to select cell types has been a challenge for the emerging field of genetic medicine, especially in diseases of the muscle," said Douglas E. Williams, President and CEO of Codiak BioSciences. "Engineered exosomes may offer a solution through their potential to selectively target muscle cells. We are excited to work with the world-class team at Sarepta to further build the engEx Platform and evaluate the potential of exosomes as next generation constructs incorporating gene therapy, gene editing and other nucleic acid payload modalities."

Under the terms of the agreement, Sarepta has the exclusive option to license Codiak's technology to develop and commercialize engineered exosome therapeutics for up to five neuromuscular targets. Sarepta and Codiak will collaborate to design exosomes that can deliver and functionally release select payloads, such as nucleic acids and gene therapy and gene editing constructs, in neuromuscular indications. If Sarepta elects to exercise its option on a target, Codiak will be responsible for research and preclinical development through IND preparation, and Sarepta will be responsible for clinical development and commercial activities. In addition to upfront, research funding and license payments, Codiak will be eligible to receive significant development and regulatory milestone payments and tiered royalties on future sales.

About engEx™ Platform
The engEx Platform is Codiak's proprietary exosome therapeutic engine for engineering and manufacturing novel exosome product candidates designed to target multiple pathways throughout the body. Using this platform, Codiak can design exosomes with precisely engineered properties, incorporate various types of biologically active molecules and direct them to specific cell types and tissues. These exosomes engage targets by cellular uptake, membrane-to-membrane interaction or a combination of both mechanisms and are designed to change the biological functioning of the recipient cells in order to produce the intended biological effect. Codiak is building a broad pipeline of engEx product candidates that may have a transformative impact on the treatment of many diseases.

About Codiak BioSciences
Codiak BioSciences is harnessing exosomes—natural intercellular messengers—to develop a new class of biologic medicines, exosome therapeutics. Utilizing our proprietary and versatile exosome engineering and manufacturing platform (engEx™ Platform), Codiak is developing exosomes with precisely engineered properties to engage pathways and deliver potent therapeutics to specific cell targets. We are building a broad pipeline of therapeutic candidates that may have a transformative impact on the treatment of many diseases, including in the areas of oncology, immune-based diseases, fibrotic disorders, neurological disorders and rare diseases. For more information, visit http://www.codiakbio.com.

About Sarepta Therapeutics
At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

Sarepta Forward Looking Statements
This press release contains "forward-looking statements." Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements regarding the parties' obligations and responsibilities under the agreement, potential payments and fees, potential neuromuscular targets and Sarepta's option to any of the candidates developed pursuant to the research alliance; the potential benefits of the engineered exosome approach, including effectively delivering genetic therapeutics without triggering the adaptive immune response and selectively targeting muscle cells; the potential of exosomes to provide a unique advantage as a targeted delivery system for genetic medicines; and the potential benefits of the collaboration between Sarepta and Codiak, including the development of next generation therapeutics for patients with neuromuscular diseases that have few or no treatment options.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the expected benefits and opportunities related to the collaboration between Sarepta and Codiak BioSciences may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development. In particular, the collaboration may not result in any viable treatments suitable for commercialization due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreement; success in preclinical trials does not ensure that later clinical trials will be successful; Sarepta may not be able to execute on its business plans and goals, including meeting its expected or planned regulatory milestones and timelines, clinical development plans, and bringing its product candidates to market, due to a variety of reasons, many of which may be outside of Sarepta's control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover Sarepta's product candidates and the COVID-19 pandemic; and even if Sarepta's programs result in new commercialized products, Sarepta may not achieve the expected revenues from the sale of such products; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2019, and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

Contacts:

Sarepta Therapeutics
Investors: Ian Estepan, 617-274-4052, iestepan@sarepta.com
Media: Tracy Sorrentino, 617-301-8566, tsorrentino@sarepta.com

Codiak BioSciences
Kate Niazi-Sai, 617-949-5696, media@codiakbio.com