Sarepta Therapeutics and GenEdit Share Progress on Research Collaboration and Announce Agreement to Develop Gene Editing Therapeutics for Neuromuscular Diseases

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- *Collaboration combines Sarepta's proprietary gene editing technologies and GenEdit's non-viral delivery platform to create new genetic medicines for patients with neuromuscular diseases*
- *Initial results from the ongoing research collaboration have demonstrated delivery to muscle tissue after systemic administration*
- *Sarepta and GenEdit will work together to identify development candidates for further testing*

CAMBRIDGE, Mass. and SOUTH SAN FRANCISCO, Calif., Feb. 01, 2022 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), the leader in precision genetic medicine for rare diseases, and GenEdit, Inc., a developer of genetic medicines that leverage its NanoGalaxy™ polymer nanoparticle platform for tissue-selective delivery, today announced a research collaboration and option agreement under which the companies are employing GenEdit's NanoGalaxy platform and Sarepta's gene editing technology to develop gene editing therapeutics for the treatment of neuromuscular diseases. As part of the agreement, Sarepta obtains exclusive option rights to license polymer nanoparticles developed by GenEdit in the collaboration for up to four neuromuscular indications selected by Sarepta.

Initial *in vivo* results from the research collaboration between GenEdit and Sarepta have demonstrated the potential of GenEdit's polymer nanoparticles to deliver therapeutic cargo to specific muscle tissue after systemic administration to allow for targeted, non-viral systemic delivery of genetic medicines. The research collaboration and option agreement commenced in December 2020.

"We've been impressed with the diversity of GenEdit's NanoGalaxy platform and its screening and selection process, which has generated a number of distinct polymers that deliver to muscle," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics. "Sarepta is committed to the development of therapies for rare neuromuscular diseases, and we look forward to continuing to work with the team at GenEdit to advance effective gene editing-based treatments for these patients."

Gene editing has the potential to revolutionize the treatment of diseases caused by genetic mutations by permanently modifying the genes that lead to disease. Sarepta is pursuing a variety of approaches to genetic medicine, including gene editing, as a potentially curative treatment for rare neuromuscular diseases. GenEdit has demonstrated in preclinical studies that its NanoGalaxy platform can selectively deliver to different tissues a variety of functional genetic medicine cargos, including CRISPR-Cas9 ribonucleoprotein, for targeted *in vivo* gene editing.

"GenEdit has demonstrated in this collaboration and in our own studies that the NanoGalaxy platform can overcome historic challenges in the field and achieve tissue-selective delivery of a broad range of genetic medicine cargos," said Kunwoo Lee, Ph.D., co-founder and chief executive officer of GenEdit. "GenEdit is excited to continue to advance our collaboration with Sarepta and work together to identify and develop gene editing therapeutic candidates for neuromuscular diseases with the goal of having a tremendous impact on patients."

In addition to research payments, under the terms of the collaboration and option agreement, GenEdit may receive up to $57 million in near-term payments and is also eligible for significant future development, regulatory and commercial milestones and tiered royalties ranging from upper-single to low-double digits on future product sales. Additional financial details were not disclosed.

About GenEdit, Inc.

At GenEdit, our mission is to discover and develop innovative genetic medicines with targeted *in vivo* delivery. Our NanoGalaxy™ platform of non-viral, non-lipid polymer nanoparticles will enable a universe of opportunities to launch the next generation of safe and effective genetic medicines. To bring these medicines to patients, we are developing our own internal pipeline and delivering a wide variety of therapeutic payloads for our partners' programs. For more information, please visit www.genedit.com and follow us on LinkedIn and Twitter.

About Sarepta Therapeutics, Inc.

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA 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; the potential for gene editing to revolutionize the treatment of diseases caused by genetic mutations by permanently modifying the genes that lead to disease; and the potential benefits of the collaboration between Sarepta and GenEdit, including the potential of GenEdit's polymer nanoparticles to deliver therapeutic cargo to specific muscle tissue after systemic administration to allow for targeted, non-viral systemic delivery of genetic medicines, Sarepta's option to license polymer nanoparticles developed by GenEdit in the collaboration for up to four neuromuscular indications selected by Sarepta, the potential to advance effective gene

editing-based treatments for patients with rare neuromuscular diseases, and the identification of development candidates for further testing.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others: the expected benefits and opportunities related to the collaboration and agreement 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; 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, 2020, 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 the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company'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, except as required by law.

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