Sarepta Therapeutics and Selecta Biosciences Enter into Research License and Option Agreement for Selecta's ImmTOR Immune Tolerance Platform in Neuromuscular Diseases

6/18/20

– Application of ImmTOR plus Sarepta's investigational gene therapies will be evaluated for Duchenne Muscular Dystrophy and Limb-Girdle Muscular Dystrophies –

CAMBRIDGE, Mass. and WATERTOWN, Mass., June 18, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics (NASDAQ: SRPT), the leader in precision genetic medicine for rare diseases, and Selecta Biosciences, Inc. (NASDAQ: SELB) today announced that they have entered into a Research License and Option agreement granting Sarepta an option to license the rights to develop and commercialize Selecta's immune tolerance platform, ImmTOR™, for use in Duchenne muscular dystrophy (DMD) and certain limb-girdle muscular dystrophies (LGMDs). In advance of exercising its option, Sarepta will conduct research and evaluate the utility of ImmTOR to minimize or prevent the formation of neutralizing antibodies (NAb) to adeno-associated virus (AAV) in connection with the administration of Sarepta's DMD and LGMD gene therapy candidates.

Sarepta's late-stage gene therapy candidates are delivered using AAV – in particular, AAVrh74. AAVrh74 was selected because of its safety profile, superior muscle tropism, empirical demonstration of high expression, and low screen-out rate for pre-existing antibodies. Currently, however, all systemic AAV-delivered constructs are one-time therapies that cannot be re-dosed due to the robust post-administration development of NAbs specific to the AAV vector. Selecta is a leader in immune tolerance and has generated strong preclinical evidence to support the potential for re-dosing patients receiving gene therapy. Selecta has reported that in preclinical studies, when used in combination with AAV gene therapy vectors, Selecta's ImmTOR immune tolerance platform inhibits the development of NAbs to the vector, permitting re-dosing of the gene therapy. [i]

"As we build our enduring gene therapy engine, we intend not only to rapidly advance treatments for rare, life-ending diseases, but at the same time, to advance the state of genetic medicine science by continually improving the utility of gene therapy. If successful, the ability to re-dose will be an enormous leap forward in the science of gene therapy and provide invaluable benefits to patients beyond those we anticipate with one-time dosing. We are encouraged by the data generated on the ImmTOR platform and excited to join with Selecta to explore the possibility of unlocking the opportunity to safely and effectively re-dose AAV-mediated gene therapies in patients with DMD and LGMDs, if needed," said Doug Ingram, President and Chief Executive Officer, Sarepta Therapeutics.

"We are pleased to build on our already strong foundation of strategic partnerships and expand the clinical application of the ImmTOR platform into neuromuscular diseases. The ability to re-dose gene therapy addresses one of the major challenges of one-time therapies today," said Carsten Brunn Ph.D., President and Chief Executive Officer of Selecta Biosciences. "We are excited to collaborate with a leader in genetic medicine like Sarepta, and are confident that their expertise in rare diseases combined with our immune tolerance platform has the potential to enhance the long-term therapeutic benefit to patients with these debilitating conditions."

DMD is a rare, degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. LGMDs are a group of over 30 distinct diseases that cause weakness and wasting of the muscles around the hips and shoulders, eventually progressing to the arms and legs. LGMD can be caused by a single gene defect that affects specific proteins within the muscle cell, including those responsible for keeping the muscle membrane intact.

Under the terms of the research license and option agreement, Sarepta will make an initial payment to Selecta, and Selecta is eligible to receive certain pre-clinical milestone fees. If Sarepta exercises its options to enter any commercial license agreements, Selecta will be eligible for additional development, regulatory, and commercial milestone payments, as well as tiered royalties on net product sales. Additional financial details are not being disclosed.

About Selecta Biosciences, Inc.

Selecta Biosciences, Inc. is a clinical-stage biotechnology company focused on unlocking the full potential of biologic therapies based on its pioneering immune tolerance platform (ImmTOR™). Selecta is committed to utilizing ImmTOR to potentially improve the efficacy of biologics, enable re-dosing of life-saving gene therapy, and create novel immunotherapies for autoimmune diseases. Selecta's late-stage product candidate, SEL-212, is designed to be a monthly treatment for chronic refractory gout, a debilitating rare disease with a significant unmet medical need. SEL-212 consists of a combination of our ImmTOR platform co-administered with pegadricase, an enzyme designed to treat patients with symptomatic gout, refractory to standard uric acid lowering treatment. Selecta's proprietary gene therapy product candidates are in development for certain rare inborn errors of metabolism and incorporate our ImmTOR platform with the goal of addressing barriers to repeat administration. In addition to our own pipeline of core discovery and clinical candidates, Selecta has established collaborative relationships with leading biopharmaceutical companies, including Asklepios BioPharmaceutical (AskBio) for gene therapy, and Swedish Orphan Biovitrum AB (Sobi™) for SEL-212. Selecta is based in Watertown, Massachusetts. For more information, please visit www.selectabio.com.

Selecta Forward-Looking Statements:

Selecta Biosciences, Inc. ("the company"), including without limitation, the company's actions regarding the monitoring and assessment of COVID-19 on the company's operations, clinical trials and manufacturing, Sarepta's plans to evaluate its gene therapies in combination with the company's ImmTOR technology, the possibility of Sarepta exercising an option to enter into a commercial license agreement, the unique proprietary technology platform of the company and the unique proprietary platform of its partners, the potential of ImmTOR to enable re-dosing of AAV gene therapy, the ability of the company's ImmTOR platform to unlock the full potential of biologic therapies, the potential treatment applications for product candidates utilizing the ImmTOR platform in areas such as enzyme therapy and gene therapy, the novelty of treatment paradigms that Sarepta is able to develop in combination with the company's ImmTOR technology, the potential of any therapies developed by Sarepta in combination with the company's

ImmTOR technology to fulfill unmet medical needs, the company's plan to apply its ImmTOR technology platform to a range of biologics for rare and serious diseases, the ability of Sarepta's existing therapies to target the heart and skeletal muscle, expected payments to be made to the company under the Research License and Option Agreement, the potential of the ImmTOR technology platform generally and the company's ability to grow its strategic partnerships, the sufficiency of the company's cash, cash equivalents and short-term investments, and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "hypothesize," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including, but not limited to, the following: the uncertainties inherent in the initiation, completion and cost of clinical trials including their uncertain outcomes, the effect of the COVID-19 outbreak on any of the company's planned or ongoing clinical trials, manufacturing activities, supply chain and operations, the availability and timing of data from ongoing and future clinical trials and the results of such trials, whether preliminary results from a particular clinical trial will be predictive of the final results of that trial or whether results of early clinical trials will be indicative of the results of later clinical trials, the unproven approach of the company's ImmTOR technology, Sarepta's ability to research and develop therapeutic candidates using the company's ImmTOR technology, undesirable side effects of the company's product candidates, its reliance on third parties to manufacture its product candidates and to conduct its clinical trials as well as the impact of the COVID-19 outbreak on those third parties and their ability to continue their operations, the company's inability to maintain its existing or future collaborations, licenses or contractual relationships, its inability to protect its proprietary technology and intellectual property, management's ability to perform as expected, potential delays in regulatory approvals, Sarepta's ability to make up-front and milestone payments, the company's business development strategy, the availability of funding sufficient for its foreseeable and unforeseeable operating expenses and capital expenditure requirements, the company's recurring losses from operations and negative cash flows from operations raise substantial doubt regarding its ability to continue as a going concern, substantial fluctuation in the price of its common stock including stock market fluctuations that occur as a result of the COVID-19 outbreak, and other important factors discussed in the "Risk Factors" section of the company's most recent Quarterly Report on Form 10-Q, and in other filings that the company makes with the Securities and Exchange Commission. In addition, any forward-looking statements included in this press release represent the company's views only as of the date of its publication and should not be relied upon as representing its views as of any subsequent date. The company specifically disclaims any intention to update any forward-looking statements included in this press release.

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 Statement

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' undertakings under the agreement and potential payments and fees; the potential benefits of Sarepta's gene therapy product candidates; the potential of ImmTOR to enable re-dosing of AAV gene therapy; Sarepta's intention to rapidly advance treatments for rare, life-ending diseases, and to advance the state of the genetic medicine science by continually improving the utility of gene therapy; the potential of re-dosing to provide invaluable benefits to patients beyond those Sarepta anticipates with one-time dosing; the possibility of unlocking the opportunity to safely and effectively re-dose AAV-mediated gene therapies in patients with DMD and LGMDs, if needed; and the potential of the collaboration between Sarepta and Selecta to enhance the long-term therapeutic benefit to patients with these debilitating conditions.

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 between Sarepta and Selecta 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.

Selecta Contacts:
For Media:
Joshua R. Mansbach
Solebury Trout
+1-646-378-2964
jmansbach@soleburytrout.com

For Investors:
Lee M. Stern
Solebury Trout

+1-646-378-2922
lstern@soleburytrout.com

Sarepta Contacts:
Investors:
Ian Estepan, 617-274-4052
iestepan@sarepta.com

Media:
Tracy Sorrentino, 617-301-8566
tsorrentino@sarepta.com

---

[i] *Nature Communications,* October 2018.