

## Spark Therapeutics Enters into Licensing Agreement with Selecta Biosciences

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*Exclusive use of Selecta's Synthetic Vaccine Particles (SVP™) platform technology provided to Spark Therapeutics for co-administration with up to five gene therapy targets, including FVIII for hemophilia A*

*SVP may enhance gene therapies by enabling repeat dosing and mitigating other potential immune responses to an AAV capsid*

PHILADELPHIA and WATERTOWN, Mass., Dec. 05, 2016 (GLOBE NEWSWIRE) -- Spark Therapeutics (NASDAQ:ONCE) and Selecta Biosciences, Inc. (NASDAQ:SELB) today announced a license agreement that provides Spark Therapeutics with exclusive worldwide rights to Selecta's proprietary Synthetic Vaccine Particles (SVP™) platform technology for co-administration with gene therapy targets, including FVIII for hemophilia A, as well as exclusive options for up to four additional undisclosed genetic targets.

Selecta's immune tolerance SVP, including SVP-Rapamycin, is an investigational technology intended to suppress the formation of neutralizing antibodies to an adeno-associated virus (AAV) capsid when used in combination with gene therapies, without altering the therapeutic profile of the gene therapy. Neutralizing antibodies form in response to an initial administration of an AAV gene therapy and prevent effective subsequent usage. The potential ability to re-dose a gene therapy may be beneficial where a patient has not achieved a sufficient therapeutic expression of the transferred gene in the initial dose.

"Selecta's nanoparticle technology, which is undergoing preclinical testing in gene therapy, may prevent formation of neutralizing antibodies, and thus potentially enable re-dosing up to an optimal therapeutic profile by extending the reach of gene therapy to diseases that require higher doses or more extensive transduction of target cells than may be achieved through one-time dosing," said Jeffrey D. Marrazzo, chief executive officer of Spark Therapeutics. "Importantly, if proven successful, the co-administration of Selecta's technology with a gene therapy may enable repeat dosing of AAV gene therapies in both adults and pediatric patients, potentially minimizing the risk of a T-cell immune response to the capsid."

"Gene therapy is a core area of focus for Selecta; one that we believe could benefit profoundly from our immune tolerance SVP technology platform," said Werner Cautreels, Ph.D., president, CEO and chairman of Selecta. "We are excited about this license agreement with Spark Therapeutics, a recognized gene therapy leader, which accelerates the application of our SVP platform in gene therapy. Our preclinical studies in this field, together with the clinical data we have generated with SEL-212 in gout showing prevention of anti-drug antibodies, suggest that the application of our immune tolerance SVP technology to biologic therapies may greatly benefit patients with life-threatening diseases who currently lack adequate treatment options due to the occurrence of undesired immune responses."

Subject to the terms of the agreement, Spark Therapeutics will make an initial \$10 million cash payment to Selecta and purchase \$5 million of Selecta's common stock. Within 12 months of the agreement's signing, Spark Therapeutics has agreed to pay Selecta an additional \$5 million in cash and to purchase \$10 million of Selecta's common stock. Selecta will be eligible for up to \$430 million in milestone payments for each target, with up to \$65 million being based on Spark Therapeutics' achievement of specified development and regulatory milestones and up to \$365 million for specified commercial milestones. In addition, Spark Therapeutics will pay Selecta tiered mid-single to low-double-digit royalties on worldwide annual net sales of any resulting commercialized gene therapy.

The terms of this agreement do not apply to Spark Therapeutics' ongoing investigational development programs in inherited retinal diseases (IRDs), including voretigene neparvovec for the treatment of *RPE65*-mediated IRD and *SPK-7001* for choroideremia. This agreement does not impact Spark Therapeutics' ongoing Phase 1/2 trial of *SPK-9001* in hemophilia B in collaboration with Pfizer or its planned Phase 1/2 trial of *SPK-8011* in hemophilia A.

Selecta independently is applying its SVP technology to its own proprietary gene therapy programs. Selecta has obtained

an exclusive license from Massachusetts Eye and Ear to Anc80, an *in silico*-designed gene therapy vector, for Methylmalonic Acidemia and has options for additional pre-defined indications. Additionally, Selecta is advancing a proprietary gene therapy program for Ornithine Transcarbamylase Deficiency.

### **About Spark Therapeutics**

Spark Therapeutics, a fully integrated company, is striving to challenge the inevitability of genetic disease by discovering, developing, and delivering gene therapies that address inherited retinal diseases (IRDs), liver-mediated diseases such as hemophilia, and neurodegenerative diseases. Our validated platform successfully has delivered proof-of-concept data with investigational gene therapies in the retina and liver. Our most advanced investigational candidate, voretigene neparvovec, in development for the treatment of *RPE65*-mediated IRD, has received orphan designations in the U.S. and European Union, and breakthrough therapy designation in the U.S. The pipeline also includes *SPK-7001*, in a Phase 1/2 trial for choroideremia, and two hemophilia development programs: *SPK-9001* in a Phase 1/2 trial for hemophilia B being developed in collaboration with Pfizer (which also has received both breakthrough therapy and orphan product designations) and *SPK-8011*, a preclinical candidate for hemophilia A to which Spark Therapeutics retains global commercialization rights. To learn more about us and our growing pipeline, visit [www.sparktx.com](http://www.sparktx.com).

### **Spark Cautionary Note on Forward-looking Statements**

This release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the company's *SPK-FIX* program. Any forward-looking statements 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. These risks and uncertainties include, but are not limited to, the risk that the SVP nanoparticle technology used in connection with gene therapies will not produce results in humans that are similar to the preclinical results observed to date. For a discussion of other 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 "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in our Annual Report on Form 10-K, our Quarterly Reports on Form 10-Q and other filings we make with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Spark undertakes no duty to update this information unless required by law.

### **About Selecta Biosciences, Inc.**

Selecta Biosciences, Inc. is a clinical-stage biopharmaceutical company developing targeted therapies that use immunomodulators encapsulated in nanoparticles to induce antigen-specific immune responses to prevent and treat disease. Selecta's proprietary Synthetic Vaccine Particles (SVP™) technology is a highly flexible nanoparticle platform capable of incorporating a wide range of antigens and immunomodulators, allowing SVP-based products to either induce antigen-specific tolerance or activate the immune system. Selecta's focus and strategy is to leverage its SVP immune modulating platform to develop and commercialize highly differentiated life-sustaining biologic drugs that are uniquely capable of mitigating the formation of anti-drug antibodies (ADAs). Proprietary programs that use SVP-Rapamycin to enhance efficacy and safety of therapy include SEL-212, Selecta's lead Phase 2 clinical program in chronic refractory gout, and two gene therapies programs for genetic metabolic diseases. Tolerance-inducing SVP biological products also have potential applications in the treatment of allergies and autoimmune diseases. Selecta is also developing SVP product candidates that activate the immune system to prevent and treat cancer, infections and other diseases. Selecta is based in Watertown, Massachusetts, USA. For more information, please visit <http://selectabio.com>.

### **Selecta Biosciences Forward-looking Statements**

Any statements in this press release about the future expectations, plans and prospects of Selecta Biosciences, Inc. ("the company"), including without limitation, statements regarding the company's expectation about receiving payments from Spark Therapeutics under the license agreement, the progress of the Phase 1/2 clinical program of SEL-212 including the number of centers in the Phase 2 clinical trial of SEL-212 and the announcement of data, conference presentations, the ability of the company's SVP platform, including SVP-Rapamycin, to mitigate immune response and create better therapeutic outcomes, the potential treatment applications for products utilizing the SVP platform including repeat dosing

for gene therapy, any future development of the company's discovery programs in peanut allergy and celiac disease, the sufficiency of the company's cash, cash equivalents, investments, and restricted cash 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 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 SVP technology, potential delays in enrollment of patients, 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, the company's inability to maintain its existing or future collaborations or licenses, its inability to protect its proprietary technology and intellectual property, potential delays in regulatory approvals, the availability of funding sufficient for its foreseeable and unforeseeable operating expenses and capital expenditure requirements, substantial fluctuation in the price of its common stock, a significant portion of the company's total outstanding shares are eligible to be sold into the market in the near future, and other important factors discussed in the "Risk Factors" section of the company's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission, or SEC, on November 10, 2016, and in other filings that the company makes with the SEC. In addition, any forward-looking statements included in this press release represent the company's views only as of the date of this release and should not be relied upon as representing its views as of any subsequent date. The company specifically disclaims any obligation to update any forward-looking statements included in this press release.

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