Selecta Biosciences Announces New Preclinical Gene Therapy Data at the Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT)

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- *Proof of Concept for Rare Disease Methymalonic Acidemia (MMA) Gene Therapy*
- *Gene Therapy Dose Titration Enabled via SVP-Rapamycin Combination Treatment*

WATERTOWN, Mass., May 15, 2017 (GLOBE NEWSWIRE) -- Selecta Biosciences, Inc. (NASDAQ:SELB), a clinical-stage biopharmaceutical company focused on unlocking the full potential of biologic therapies by avoiding unwanted immune responses, today announced new preclinical data regarding non-immunogenic gene therapies that were presented at the American Society of Gene & Cell Therapy (ASGCT) 2017 Annual Meeting, which took place last week in Washington, D.C.

“Immunogenicity is a key challenge in gene therapy, limiting the number of diseases and patients that can be effectively treated and presenting a safety hurdle,” said Werner Cautreels, Ph.D., CEO and Chairman of Selecta. “Together with various collaborators, we have again demonstrated the potential of Selecta’s proprietary immune tolerance Synthetic Vaccine Particles (SVP™) technology, which is designed to improve the clinical benefits and transform the development of gene therapy. We also were pleased with the presentation of preclinical proof-of-concept data for our proprietary product candidate in MMA, a life-threatening rare disease that can only be treated today by diet or organ transplantation.”

A team led by Charles Venditti, M.D., Ph.D., Senior Investigator and Head, Organic Acid Research Section in the National Human Genome Research Institute at the National Institutes of Health, and Luk Vandenberghe, Ph.D., Director of the Grousbeck Gene Therapy Center at Mass. Eye and Ear and an Assistant Professor at Harvard Medical School, delivered a presentation entitled “Anc80 Mediates Hepatic Correction of Methylmalonyl-CoA Mutase Deficiency in Murine Models of Methymalonic Acidemia.” This presentation featured data from mouse models of MMA, a rare inborn error of metabolism most frequently caused by mutations in the enzyme methylmalonyl-CoA mutase (MUT). In this study, MUT-deficient mice were treated with Selecta’s Anc80-synMUT product candidate to express the human MUT gene. The gene therapy induced a robust biochemical and clinical response as plasma methylmalonic acid levels dropped precipitously, substantial weight gain ensued and survival was sustained. Further, presented data indicate that the combination of SVP and Anc80 could effectively overcome the immunogenicity that has limited other gene therapy programs by enabling enrollment of patients with pre-existing antibodies to AAV and keeping patients eligible for repeat administration.

A team led by Federico Mingozzi, Ph.D., Head of Immunology and Liver Gene Therapy at Genethon, delivered a presentation entitled “Modulation of AAV Vector Dosing and Avoidance of Capsid Immune Responses via Repeated Co-Administration of Vector with Rapamycin Tolerogenic Nanoparticles.” This presentation featured data from both mouse and non-human primate studies demonstrating how the co-administration of SVP-Rapamycin completely blocked anti-AAV immune responses in an antigen-specific manner and allowed for vector re-administration and gene therapy dose titration. The ability to dose titrate could provide for more effective development and administration of gene therapies.

Click here to view these presentations.

About Selecta's MMA Program

MMA is an inborn error of metabolism that, according to the U.S. National Institutes of Health (NIH), affects an estimated one in 25,000 to 48,000 individuals globally. MMA patients are unable to process certain proteins and fats, leading to the accumulation of toxic metabolites. Symptoms of this life-threatening disease start to develop in early childhood and, despite strict diet, patients suffer from a wide range of disease-related complications such as pancreatitis, strokes and chronic kidney failure. Selecta exclusively licensed Anc80 for MMA from Massachusetts Eye and Ear® (MEE) in May 2016. Under the license agreement, Selecta also has the exclusive option to develop gene therapies using

Anc80 for additional pre-defined lysosomal storage, genetic muscular and genetic metabolic diseases. In early 2017, Selecta entered into a strategic manufacturing agreement with Lonza Houston, Inc. under which Lonza will produce an Anc80-AAV-based gene therapy product for Selecta's MMA program.

Selecta intends to combine Anc80 with recently discovered transgenes and Selecta's SVP-Rapamycin to create a novel gene therapy for MMA. This therapy is intended to a) enable the treatment of patients with and without pre-existing anti-AAV antibodies; b) prevent cellular immune responses that often reduce the expression levels of gene therapies; and c) provide the ability to administer repeat gene therapy doses to achieve sufficient levels of methylmalonyl-CoA mutase (MUT), the enzyme that MMA patients are lacking.

To advance the MMA program, Selecta entered into a Collaborative Research and Development Agreement (CRADA) with MEE and the National Human Genome Research Institute, NIH, in 2016. Principal investigators in this CRADA initiative are Charles Venditti, M.D., Ph.D., Senior Investigator and Head, Organic Acid Research Section in the National Human Genome Research Institute at the National Institutes of Health, and Luk Vandenberghe, Ph.D., Director of the Grousbeck Gene Therapy Center at MEE and an Assistant Professor at Harvard Medical School. A physician-scientist specializing in the study of inborn errors of metabolism including MMA, Dr. Venditti and his group have published several studies showing the effectiveness of gene therapy as a treatment for MMA in mice. Dr. Vandenberghe from MEE is the inventor of Anc80.

About Selecta Biosciences, Inc.

Selecta Biosciences, Inc. is a clinical-stage biopharmaceutical company that is focused on unlocking the full potential of biologic therapies by avoiding unwanted immune responses. Selecta plans to combine its tolerogenic Synthetic Vaccine Particles (SVP™) to a range of biologics for rare and serious diseases that require new treatment options. The company's current proprietary pipeline includes SVP-enabled enzyme, oncology and gene therapies. SEL-212, the company's lead candidate in Phase 2, is being developed to treat severe gout patients and resolve their debilitating symptoms, including flares and gouty arthritis. Selecta's clinical oncology candidate, LMB-100, is in a Phase 1 program targeting pancreatic cancer and mesothelioma. Its two proprietary gene therapy product candidates are being developed for rare inborn errors of metabolism and have the potential to enable repeat administration. The use of SVP is also being explored in the development of vaccines and treatments for allergies and autoimmune diseases. Selecta is based in Watertown, Massachusetts. For more information, please visit http://selectabio.com and follow @SelectaBio on Twitter.

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, whether the company's MMA product candidate will prevent cellular immune responses, enable repeat administration or allow for the treatment of patients with and without pre-existing anti-AAV antibodies, the company's ability to unlock the full potential of biologic therapies, the company's plan to apply its SVP platform to a range of biologics for rare and serious diseases, the potential of SEL-212 to treat severe gout patients and resolve their debilitating symptoms, the potential of the company's two gene therapy product candidates to enable repeat administration, the potential treatment applications for products utilizing the SVP platform in areas such as gene therapy, immuno-oncology, allergies, autoimmune diseases and vaccines, 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 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 May 11, 2017, 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 its publication 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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