Exclusive License Anc80 Gene Therapy Vector

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Selecta Biosciences Obtains Exclusive License to Proprietary Gene Therapy Vector from Massachusetts Eye and Ear

- *Selecta is licensing Anc80 for a rare genetic disease with options on additional pre-specified indications*
- *By combining Anc80 with Selecta's investigational drug candidate, SVP-Rapamycin (SEL-110), Selecta is seeking to advance a new gene therapy platform designed to avoid several immunogenicity challenges that limit the development of gene therapies today*

Watertown, Mass. – May 19, 2016 – Selecta Biosciences, Inc., a clinical-stage biopharmaceutical company developing targeted antigen-specific immune therapies, announced today that it obtained an exclusive license to Anc80, an *in silico* designed gene therapy vector, from Massachusetts Eye and Ear. Under the agreement, Selecta secures an exclusive license to the Anc80 technology for a rare genetic disease and has options for additional pre-defined indications in the areas of lysosomal storage diseases, genetic muscular diseases and genetic metabolic diseases. Anc80 is developed by the laboratory of Luk H. Vandenberghe, PhD, Director of the Grousbeck Gene Therapy Center at Mass. Eye and Ear and an Assistant Professor at Harvard Medical School. Anc80 has demonstrated the potential to provide superior gene expression levels in the liver compared to naturally-occurring adeno-associated viral vectors (AAVs) that are currently in clinical development. Anc80, a next generation engineered gene therapy vector, is synthetic in nature and has been shown to have reduced cross-reactivity with commonly used AAV vectors.

By combining Anc80 with Selecta's investigational drug candidate SVP-Rapamycin (SEL-110), Selecta is seeking to advance a new gene therapy platform designed to avoid immunogenicity that restricts the application of gene augmentation, replacement and editing therapies. Gene therapy vectors are derived from viruses and are designed to insert a therapeutic transgene into the nucleus of targeted cells. Undesired immunogenicity can manifest itself in three ways. First, pre-existing neutralizing anti-drug antibodies (ADAs) induced following a natural AAV infection can block gene transfer. Today up to 50 percent of patients are ineligible for gene therapy due to the presence of pre-existing ADAs. Second, ADAs form in response to a first administration of a gene therapy vector and prevent effective subsequent doses of gene therapy. The ability to re-administer is important for therapeutic targets that undergo renewal which is the case in many pediatric indications. Lastly, cellular immune responses against the transduced cells can reduce efficacy and pose safety concerns. Selecta intends to combine Anc80 with SVP-Rapamycin (SEL-110), a proprietary immunomodulatory therapeutic, with the objective to address all three of these immunogenicity issues related to gene therapies.

"The combination of Anc80 with SVP-Rapamycin is very promising," said Dr. Vandenberghe. "Anc80 is a potent gene therapy vector that is not known to circulate in humans, making it less likely to cross-react immunologically with naturally occurring AAVs. Selecta's SVP-Rapamycin has shown the potential to mitigate the immune responses that are connected with the administration of viral vectors. The combination of Anc80 with SVP-Rapamycin offers the exciting prospect of broadening the potential of gene therapy in both the proportion of patients it can reach and the number of diseases amenable to this emerging therapeutic modality in medicine."

"This agreement provides Selecta with the possibility of developing several proprietary gene therapies using a novel and differentiated vector," said Werner Cautreels, PhD, CEO and President of Selecta. "The agreement with Massachusetts Eye and Ear is a cornerstone in our strategy to build a pipeline of proprietary products to treat rare and serious diseases by mitigating the immune responses that would otherwise compromise these therapies. In addition to gene therapy, our efforts are focused on the mitigation of deleterious immune responses to otherwise highly effective therapeutic enzymes and proteins."

Selecta intends to develop the combination of Anc80 and SVP-Rapamycin (SEL-110) to increase the potential applicability of gene therapies. This would include (i) patients with pre-existing ADAs to naturally occurring AAV, a current exclusion criterion for many clinical studies, and (ii) diseases that require repeat dosing due to a young patient population or the need to reach higher levels of protein expression than can be achieved with a single dose.

When co-administered with gene therapy vectors in animal models, Selecta's SVP-Rapamycin (SEL-110) has been observed to mitigate the formation of ADAs as well as inflammatory immune responses that are associated with the first dose of a gene therapy vector. The product is currently being studied in a clinical trial to mitigate undesired immune responses to pegsiticase, an enzyme used for the treatment of gout.

About Anc80

Developed by the laboratory of Luk H. Vandenberghe, PhD, of Mass. Eye and Ear and Harvard Medical School, Anc80 is an *in silico* designed predicted ancestor of AAV1, AAV2, AAV8 and AAV9. In preclinical studies, Anc80 has been shown to be a potent gene therapy vector that has demonstrated the capability of yielding superior gene expression levels in the liver. As a synthetic vector, Anc80 has reduced pre-existing ADAs and limited cross-reactivity to naturally-occurring AAVs. Dr. Vandenberghe is a co-inventor on several AAV vector technologies and methods, including Anc80, which are licensed to biotech and pharmaceutical entities for which he receives royalties.

About Massachusetts Eye and Ear

Mass. Eye and Ear clinicians and scientists are driven by a mission to find cures for blindness, deafness and diseases of the head and neck. Now united with Schepens Eye Research Institute, Mass. Eye and Ear is the world's largest vision and hearing research center, developing new treatments and cures through discovery and innovation. Mass. Eye and Ear is a Harvard Medical School teaching hospital and trains future medical leaders in ophthalmology and otolaryngology, through residency as well as clinical and research fellowships. Internationally acclaimed since its founding in 1824, Mass. Eye and Ear employs full-time, board-certified physicians who offer high-quality and affordable specialty care that ranges from the routine to the very complex. In the 2015–2016 "Best Hospitals Survey," *U.S. News & World Report* ranked Mass. Eye and Ear #1 in the nation for ear, nose and throat care and #1 in the Northeast for eye care. For more information about life-changing care and research, or to learn how you can help, please visit MassEyeAndEar.org.

About Selecta

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 Particle (SVP) technology is a highly flexible nanoparticle platform, capable of incorporating a wide range of antigens and immunomodulators, allowing the SVP products to either induce antigen-specific tolerance or activate the immune system.

Selecta's focus is on developing and commercializing differentiated therapies that are designed to modulate the immune system to effectively and safely treat rare diseases by mitigating the formation of anti-drug antibodies (ADAs) in response to life-sustaining biologic drugs. Tolerance-inducing SVP products also have potential applications in the treatment of allergies and autoimmune diseases.

Selecta is also developing SVP products that activate the immune system to prevent and treat cancer, infections and other diseases.

Selecta is based in Watertown, Massachusetts, USA.

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