



## Selecta and Genethon Collaborate to Create Next Generation Gene Therapies Using Selecta's Synthetic Vaccine Particle Platform

*Collaboration to pioneer first-of-a-kind gene therapies enabling multiple dosing*

**Watertown, Mass., USA, and Evry, France – May 13, 2015** – [Selecta Biosciences, Inc.](#) and [Genethon](#) today announced an ongoing research collaboration with the goal of enabling repeat dosing for gene therapies. Based on preliminary results, the companies have identified three applications that might benefit from combining Genethon's expertise in the development of gene therapy vectors and Selecta's Synthetic Vaccine Particle (SVP™) platform to prevent undesired immune responses. The companies plan to co-develop and co-own these next generation gene therapies, each with the potential to meet significant unmet patient needs.

Under the terms of the proposed collaboration, Selecta and Genethon will apply Selecta's SVP platform in an effort to eliminate the neutralizing antibodies and other undesired immune responses to the viral vector used in gene therapy. The combination of Genethon's novel gene therapies with Selecta's proprietary SVP would, for the first time, allow repeated systemic dosing of gene therapy vectors. Selecta's SVP platform has unique capabilities to engineer nanoparticles with the structure and composition to produce targeted immune tolerance by attenuating the undesired immune response specifically to viral vectors. Using SVP offers the potential to expand the therapeutic range for gene therapies by maintaining the efficacy of the gene therapy over several doses. This is of particular interest in children, where organs that produce the gene therapy products are growing, and in applications where high amounts of proteins need to be supplied by the gene therapy.

"Gene therapies that can be applied repeatedly would exponentially increase the number of highly beneficial applications of gene therapies including muscular dystrophies and pediatric liver metabolic diseases," says Fulvio Mavilio, PhD, Scientific Director of Genethon. "I'm excited by the tremendous potential of combining Genethon's gene therapies with Selecta's novel SVP platform."

"Through this collaboration with Genethon we are pioneering new ground, aiming to enable, for the first time, the ability to allow repeated systemic dosing of gene therapy vectors that can open many new disease applications for patients, particularly children," said Peter Keller, Selecta's Chief Business Officer. "Our collaboration with Genethon, a leader in gene therapies, is a good example of the value that antigen-specific SVP immunotherapies can create in novel classes of biologics and co-development deal structures could serve as a template for other SVP applications in such biologic therapies."

Genethon and Selecta will initially focus their collaborative research and co-development efforts on gene therapies in development for muscular dystrophies and pediatric liver metabolic diseases that employ adeno-associated virus (AAV) vectors, which are a gene transfer platform of choice for many *in vivo* therapy applications. In recent years, the field of *in vivo* gene transfer with AAV vectors has seen a

dramatic expansion in the number of indications approaching or in clinical testing, including promising results obtained in clinical trials of AAV vector mediated gene therapy for hemophilia B, Leber's congenital amaurosis, and others, along with the market approval of an AAV gene therapy drug for the treatment of lipoprotein lipase deficiency in Europe.

#### **About Genethon**

Généthon, created by the AFM-Telethon, has the mission to make innovative gene therapy treatments available to patients affected with rare genetic diseases. Having played a pioneering role in deciphering the human genome, Généthon is today, with more than 200 scientists, physicians, engineers and regulatory affairs specialists, one of the leading organizations for the development of gene therapy treatments. Généthon has also built one of the largest sites worldwide for GMP production of gene therapy products, Généthon Bioprod. In 2012, Généthon was awarded the prestigious Prix Galien for Pharmaceutical Research (France) In 2015, Genethon was one of 16 winners of the World Innovation Competition 2030 for its project on the development of an industrial process for production of gene therapy vectors.. The pipeline of Généthon includes products currently in international clinical trials and at preclinical stages, for immune deficiencies, muscular dystrophies, ocular and liver diseases. These products are developed either with Généthon as sponsor, or in partnership with private companies and academic institutions.

#### **About Selecta**

[Selecta Biosciences](#), Inc. is a clinical-stage biotechnology company developing novel drugs that use immune modulating nanomedicines to generate targeted antigen-specific immune responses to prevent and treat disease. Selecta's proprietary Synthetic Vaccine Particle ([SVP](#)) platform creates a novel paradigm in immunotherapeutics and vaccines, enabling completely new applications while offering the potential of improved efficacy and safety profiles.

Selecta's [immunomodulatory SVPs](#) can induce antigen-specific immune tolerance, enabling them to be applied in a variety of therapeutic areas with large unmet medical need. The company is focused on three key near-term applications: inhibition of immunogenicity of biologic therapies, treatment of allergies, and treatment of autoimmune diseases. Immunogenicity adversely affects the safety and efficacy profile for many biological therapies, and is known to have caused the termination of a number of promising biological therapies in clinical development. Selecta's SVP is a product engine that has the potential to unlock the full therapeutic value of biologic therapies.

Through proprietary products and collaborations with leading pharmaceutical companies and research organizations, Selecta is building a [pipeline](#) of product candidates to address unmet medical needs in serious and chronic diseases. Selecta Biosciences, Inc. is based in Watertown, Massachusetts, USA. For more information, please visit [www.selectabio.com](http://www.selectabio.com).

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