



Généthon and CRISPR Therapeutics announce Research Collaboration

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– Généthon, a leader in the field of gene therapy treatments for rare diseases, and CRISPR Therapeutics, a biopharmaceutical company focused on developing transformative gene-based medicines for patients with serious diseases, have today announced an ongoing research collaboration. Généthon is supporting the development of one CRISPR Therapeutics' undisclosed propriety programs. Généthon's expertise is in the pre-clinical development, clinical development and production of gene therapy products.

"We are pleased to work with one of the leading companies in the exciting field of CRISPR-Cas9 gene editing. Our pioneering expertise in designing, developing, and moving innovative gene-based medicines to the clinic makes this collaboration an excellent strategic fit for both organizations. Généthon will help to accelerate gene therapy research and innovative translational applications for rare diseases to the benefit of patients," said Fulvio Mavilio, Ph.D., Scientific Director of Généthon.

"As we continue to enter into new partnerships, we remain focused on developing transformative gene-based medicines for serious human diseases," said Bill Lundberg, M.D., Chief Scientific Officer of CRISPR Therapeutics. *"We are pleased to establish this partnership with Généthon to accelerate the delivery of these promising therapies to patients with serious diseases."*

About Généthon

Généthon, created by the AFM-Telethon, is dedicated to making 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. www.genethon.com

About CRISPR Therapeutics

The mission of CRISPR Therapeutics is to develop transformative gene-based medicines for patients with serious diseases. Our therapeutic approach is to cure diseases at the molecular level using the breakthrough gene editing technology called CRISPR-Cas9. With our multi-disciplinary team of world-renowned academics, drug developers and clinicians, we are uniquely positioned to translate CRISPR-Cas9 technology into human therapeutics. We have licensed the foundational CRISPR-Cas9 patent estate for human therapeutic use from our scientific founder, Dr. Emmanuelle Charpentier. We are headquartered in Basel, Switzerland, our R&D operations are in Cambridge, Massachusetts and we have corporate offices in London, United Kingdom. www.crisprtx.com

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