CRISPR Therapeutics and Anagenesis Biotechnologies Announce Strategic In-Licensing and Collaboration Agreement to Develop CRISPR/Cas9-based Cell Therapies for Muscle Diseases

CRISPR Therapeutics Gains Exclusive Worldwide License to Proprietary Paraxial Mesoderm Multipotent Cells (P2MCs) Technology with Initial Research Focused on Duchenne Muscular Dystrophy

BASEL, Switzerland and ILLKIRCH GRAFFENSTADEN, France– June 8, 2016 – CRISPR Therapeutics and Anagenesis Biotechnologies today announced a strategic in-licensing and collaboration agreement, which grants CRISPR Therapeutics exclusive worldwide license to Anagenesis' proprietary Paraxial Mesoderm Multipotent Cells (P2MCs) technology for cell therapy for all human muscle diseases. The P2MC technology allows for the efficient, reproducible and chemically defined differentiation of pluripotent cells into skeletal muscle stem cells, also known as satellite cells. The agreement will support the advancement of CRISPR-based cellular therapies for the treatment of musculoskeletal diseases. Initial research will focus on Duchenne Muscular Dystrophy (DMD).

"We are highly committed to finding new treatments for DMD and this collaboration is an important building block in support of our broader research strategy within DMD and other musculoskeletal diseases," said Samarth Kulkarni, Chief Business Officer of CRISPR Therapeutics.

Anagenesis' proprietary P2MC technology was developed with the support of AFM-telethon, INSERM-Transfert, CNRS and Université de Strasbourg. This technology, in combination with CRISPR/Cas9 gene editing, has the potential to yield important new treatments for boys with DMD, and for patients with other muscle disorders.

“Bringing together the CRISPR gene editing platform with the P2MC technology enables us to develop *ex vivo* therapeutic approaches for the treatment of DMD using muscle satellite stem cells,” said Bill Lundberg, M.D., Chief Scientific Officer of CRISPR Therapeutics.

“We are excited to partner with CRISPR Therapeutics, a leader in the field of CRISPR gene editing, to advance therapeutic development for musculoskeletal diseases, including DMD, where there is a high unmet medical need,” said Jean-Yves Bonnefoy, PhD, President and Chief Executive Officer of Anagenesis. “We believe our P2MC technology has a unique advantage and provides strong synergy with CRISPR Therapeutics’ gene editing technology. This partnership will be a cornerstone for our newly formed cell therapy-focused US subsidiary, Anagenesis Biotherapies Inc., located in Boston.”

Anagenesis is the third collaboration for CRISPR Therapeutics in the past seven months, and follows agreements with Vertex Pharmaceuticals and Bayer AG. Each collaboration allows the company to access distinctive capabilities to bring new, potentially transformative gene-based medicines to patients with serious diseases. As part of this collaboration, Olivier Pourquié, PhD, scientific founder of Anagenesis and professor at Harvard Medical School and the Brigham and Women’s Hospital, will serve as a consultant to CRISPR Therapeutics.

About CRISPR Therapeutics

CRISPR Therapeutics is a leading gene editing company focused on the development of transformative gene-based medicines for serious diseases using its proprietary CRISPR/Cas9 gene editing platform. CRISPR is a revolutionary technology that allows for precise, directed changes to genomic DNA. The Company’s multi-disciplinary team of world-class researchers and drug developers is working on a number of wholly-owned programs to treat serious diseases with high unmet need. Additionally, CRISPR Therapeutics is working on a number of additional programs in partnership with some of the world’s leading pharmaceutical and biotechnology companies to translate this technology into breakthrough human therapeutics. The foundational CRISPR/Cas9 patent estate for human therapeutic use was licensed from scientific founder Emmanuelle Charpentier, Ph.D., who co-invented the application of CRISPR/Cas9 for gene editing. CRISPR Therapeutics is headquartered in Basel, Switzerland with its R&D operations based in Cambridge, Massachusetts. For more information, please visit www.crisprtx.com.

About Anagenesis Biotechnologies

Anagenesis Biotechnologies is a private company developing new treatments against muscle diseases (genetic such as DMD and chronic such as sarcopenia and cachexia). The company was cofounded by Dr. Olivier Pourquié, a worldwide key opinion leader in the field of musculoskeletal development and stem cells. Olivier Pourquié is a Professor at Harvard Medical School and the Brigham and Women's Hospital and a member of the Harvard Stem Cell Institute. Anagenesis Biotechnologies is backed by a solid, experienced team led by its President & CEO, Dr. Jean-Yves Bonnefoy, coming from the Pharma & Biotech industry. Anagenesis Biotechnologies in Illkirch, France, is now focusing on HTS and HCS screens, while its newly formed US-subsidiary in Boston, MA, Anagenesis Biotherapies Inc., is developing the cell therapy approaches. For more information, please visit www.anagenesis-biotech.com.

MEDIA CONTACTS:

Jennifer Paganelli
W2O Group for CRISPR
347-658-8290
jpaganelli@w2ogroup.com

Jean-Yves Bonnefoy
Anagenesis
+33 620 885 092
jybonnefoy@anagenesis-biotech.com