Anagenesis Biotechnologies and Ksilink announce a collaborative research agreement to apply stem-cell derived disease models of Duchenne Muscular Dystrophy to small molecule therapies discovery and development

Strasbourg, France, February 2nd, 2016. Anagenesis Biotechnologies and Ksilink announced today the signature of a collaborative research agreement. Each partner will apply its expertise to the discovery and development of small molecule therapies for Duchenne Muscular Dystrophy (DMD) thanks to unique stem-cell derived disease models.

A one-of-a-kind collaboration to develop new therapies against DMD

Anagenesis Biotechnologies develops a proprietary technology that allows for the efficient, reproducible and chemically defined differentiation of pluripotent cells into mature skeletal muscle. This unique achievement was widely acknowledged with the publication of these results in the renowned scientific journal *Nature Biotechnology*. Interestingly, the company has shown that differentiated embryonic stem (ES) cells derived from *mdx* mice exhibit a striking branched phenotype resembling that described in DMD patients, thus providing an attractive model to study the origin of the pathological defects associated with DMD. This work therefore opens the possibility to develop *in vitro* models to study the pathology of muscular dystrophies as well as high throughput assays based on myogenic cells for drug screening.

Ksilink is a public private partnership that spans an integrated therapy development process from "bed to the bench, and back to the bed". Having access to leading clinical and life science expertise in France and Germany, Ksilink sources the most effective strategies towards patient needs. It uses cutting edge technologies to de-risk the science underlying the development path, specializing on the use of patient induced pluripotent stem cells (iPSC)-based disease models. Ksilink also functions as a program-specific investment fund enabling partners to carry innovation to the patient.

With this collaborative research agreement, both partners will take advantage of Anagenesis Biotechnologies' proprietary skeletal muscle system and of Ksilink's expertise and equipment to identify the phenotypic differences between DMD and wild type (WT) cells differentiated *in vitro*, and screen for compounds capable of "rescuing" the DMD cells back to a WT phenotype. Once identified, the hits will go through selection and optimization stages, with the aim of initiating clinical trials within 5 years with the compound showing most promise as a drug candidate against DMD.

Jean-Yves Bonnefoy, Anagenesis Biotechnologies' President and CEO, said "Our disease modeling technology finds in Ksilink an ideal partner to identify drugs for DMD patients, thanks to a very original and powerful high content screening approach."

Ulf Nehrbass, Ksilink's CEO, said "Anagenesis' leadership in DMD disease modeling for the first time allows for a clear and systematic path towards a target-free pharmacological rescue of muscle

cells in DMD patients. It is an exciting opportunity to put recent advances in technology into the service of patient. Together, we break new ground here."

About Duchenne Muscular Dystrophy

Duchenne Muscular Dystrophy (DMD) is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500 boys born worldwide. A devastating and incurable muscle-wasting disease, DMD is associated with mutations in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness in the lower limbs spreads to the arms, neck and other areas. Eventually, increased difficulty in breathing due to respiratory muscles dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and death usually occurs before the age of 30.

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 Pr. 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 Woman'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. Jean-Yves comes from the Pharma & Biotech industry and brings to the team his experience in the field of pharmaceutical development. For more information: www.anagenesis-biotech.com

About Ksilink

Ksilink is a public-private initiative based on the partnership of seven French and German members (Sanofi, Inserm, DKFZ, Mannheim University, Alsace Biovalley, BioPro and Strasbourg University). It is led by Dr. Ulf Nehrbass. Ulf Nehrbass has spent his recent career at Institut Pasteur in Paris and in its international network. As the Director and founder of Institut Pasteur Korea he has been invested in the application of basic research towards therapy development.

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