



Anagenesis Biotechnologies technology rewarded by the international scientific community through publication in *Nature Biotechnology*

Illkirch, France, 3rd of August 2015. Anagenesis Biotechnologies announced today a key publication in *Nature Biotechnology*. The article is entitled “Differentiating embryonic stem cells to muscle fibers to model Duchenne Muscular Dystrophy” by Jérôme Chal *et al.* from the lab of Pr Olivier Pourquié (the two scientists inventors of Anagenesis Biotechnologies technology) together with co-authors from Anagenesis Biotechnologies.

Scientific Findings published in *Nature Biotechnology*

Key cell types including skeletal muscle have proven difficult to differentiate *in vitro* from pluripotent cells. By recapitulating the development of skeletal muscle in chemically defined conditions, we have shown that myogenic cells can be produced *in vitro* from mouse and human embryonic (ES) and reprogrammed (iPS) stem cells without genetic manipulations or cell sorting. Our technology allows for efficient production of striated contractile muscle fibers and of Pax7-positive cells with satellite cell characteristics. The satellite-like cells produced *in vitro* were able to generate dystrophin-positive fibers when grafted into muscles from dystrophin-deficient *mdx* mice *in vivo*, thus providing a first proof of concept for cell therapy protocols for Duchenne Muscular Dystrophy (DMD). Moreover we showed that differentiated ES cells derived from *mdx* mice exhibit a striking branched phenotype resembling that described *in vivo*, thus providing an attractive model to study the origin of the pathological defects associated with DMD. This work also 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.

Olivier Pourquié, Professor of Genetics, Harvard Medical School, said “Muscle fibers presenting such a high level of organization and maturation have never been produced *in vitro*. We have now an ideal tool to explore the impact of dystrophin mutations on the development of muscle fibers obtained from iPS cells from DMD patients and to test therapeutic strategies aiming at correcting the pathology. Also, we can now produce satellite cells *in vitro*, which are the muscle stem cells, from human pluripotent cells. These cells have the highest regenerative potential *in vitro* and are thus an ideal candidate for grafting in cell therapies approaches for DMD.”

Jean-Yves Bonnefoy, Anagenesis Biotechnologies’ President and CEO, said “I am delighted to see such a scientific recognition for the outstanding work of Jérôme, Olivier and co-workers and as a consequence, of Anagenesis Biotechnology technology in *Nature Biotechnology*. Moreover, the peer-reviewed data validate the “proof of concept” of our technology and confirms its unique potential for our drug development programs for muscular diseases, including DMD”.

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 company that develops new treatments against muscle diseases (genetic such as DMD and chronic such as sarcopenia and cachexia) which goal is to preserve/give people autonomy, freedom to move, dignity and social life. Our mission is based on a vision shared by all stakeholders at Anagenesis Biotechnologies: to create a world where everyone can move, every single day. 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. Olivier Pourquié and Anagenesis Biotechnologies have both benefited from the long standing support from AFM-Téléthon patient association. 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

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