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Takeda and UCL to work together to tackle muscle disorders

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Japanese pharmaceutical company Takeda will work with University College London (UCL) to drive research into tackling muscle disorders, in particular muscular dystrophy.

The research – which is being conducted by the research group of Dr Francesco Saverio Tedesco – is being supported through funding of $250,000 from the company's New Frontier Sciences group. Takeda's NFS aims to support innovative, cutting-edge research which could eventually lead to drug discovery and development.

Dr Tedesco's team will focus on the study of muscular regeneration and the potential for stem cell therapies to treat muscular dystrophy, in particular induced pluripotent (iPS) stem cells.

The team is also investigating the potential for treating muscular dystrophy through developing novel gene and cell therapy strategies using artificial human chromosomes and novel biomaterials.

Using this approach, Dr Tedesco hopes to overcome a number of current limitations to developing effective treatments for muscular dystrophies. It is hoped that through the use of these modified stem cells, large quantities of progenitor cells could be produced to be transplanted into a patient's muscle following genetic correction or to be used for drug development platforms.

Importantly, the team will attempt to produce these cells which can be

applied more easily in a clinical context, in order to reduce the hurdles that might limit their possible future use in clinical studies.

Through previous work using a mouse model of Duchenne muscular dystrophy, the team has already demonstrated the potential of pre-clinical gene replacement therapy using an artificial human chromosome.

Moreover, in a separate study, Dr Tedesco and his team also demonstrated the potential of genetically corrected iPS cells which had been transplanted into another mouse model of a genetic muscle disorder (limb-girdle muscular dystrophy 2D).

Commenting on the new funding for his research, Dr Tedesco, from the UCL Department of Cell and Developmental Biology, said:

"This funding from Takeda comes at a crucial time for building on our research to develop new and innovative approaches to developing potentially life-changing treatments for muscular dystrophies.

"With Takeda's backing, my team and I will be building on the processes we've already shown to be promising and which could pave the way for the development of novel strategies for both cell therapies and drug discovery in muscle disorders."

Gordon Wong, D.Phil., Head of New Frontier Science at Takeda, added:

"We are delighted to support Dr Tedesco and his team because their ground breaking work has the potential for significant patient benefit."

"That several different strands of their research have already borne fruit was strong evidence for us of the translational potential of Dr Tedesco's research for muscular dystrophies."