Clinical trial offers hope for young men with muscular dystrophy

A gene therapy clinical trial begins this week which could offer new hope to muscular dystrophy sufferers.

A new treatment called molecular patch therapy has been developed which has the potential to give boys born with Duchenne Muscular Dystrophy (DMD) the chance to preserve their muscle function and live into old age.

In a world first the antisense oligonucleotide (AO) patches work by masking the faulty part of the gene (exon 51) and allowing shortened but functional proteins to be formed.

DMD affects one in 3,500 boys and is caused by reduced production of dystrophin protein - vital for muscle function. The progression of the condition is so severe that untreated boys lose the ability to walk by their early teens are only expected to live into their twenties.

“This is a major breakthrough for the treatment of DMD” said Professor Francesco Muntoni, head of the neuromuscular unit at Imperial College Helathcare NHS Trust. “As conventional gene therapy approach for this disorder has proven to be problematic. Animal work has suggested that the molecular patch has worked well and showed a very significant restoration in dystrophin function”.

Professor Muntoni leads the MDEX Consortium, a multidisciplinary enterprise promoting translational research into muscular dystrophies, and is formed by the clinical groups of Professor Francesco Muntoni (Imperial College London) and Professor Kate Bushby and Professor Volker Straub (Newcastle University), and scientists from Imperial College London (Professor Dominic Wells, Dr Jennifer Morgan), Royal Holloway University of London (Professor George Dickson, Dr Ian Graham) and Oxford University (Dr Matthew Wood). In addition the charities Muscular Dystrophy Campaign (MDC), Parent Project UK (PPUK) and Duchenne Parent Support Group also participate in the Consortium.

The Consortium has been responsible for generating and testing multiple molecular patches to find the optimal one to be used in this trial. The molecular patches were tested in cell cultures and the patch that restored the highest level of dystrophin protein is now being taken to clinical trial.

The phase one clinical trial is due to start by the end of October at two of Imperial College Healthcare NHS Trust’s sites - Hammersmith Hospital and at the new Paediatric Research Unit at St Mary’s Hospital.

It will recruit nine young men aged 12-17 with DMD who will have the molecular patch administered by injection into a small muscle in the foot. Subject to the trial’s success, there is already a plan to proceed with another trial to deliver the molecular patch under the skin, so that all muscles in the body could be treated.

The Consortium has attracted a large grant from the Department of Health (£1.6 million) which is funding the present study. Recent additional funds from the Medical Research Council (MRC), MDC, PPUK, Big Lottery Fund (BLF), Parent Project Muscular Dystrophy (PPMD) and Muscular Dystrophy Ireland (MDI) will allow the Consortium to take the current studies forward.
The molecular patch design is in collaboration with Professor Steve Wilton (Perth, Australia). Trial support and clinical grade molecular patches are being provided by AVI BioPharma (Portland, Oregon, USA).

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Notes to Editors

The Imperial College Healthcare NHS Trust comprises Charing Cross, Hammersmith Hospital, Queen Charlotte’s & Chelsea, St Mary’s and Western Eye hospitals. It is the largest Trust in the country, and in partnership with Imperial College London, is the UK’s first Academic Health Science Centre (AHSC).

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