MEDIA ANNOUNCEMENT

TBC 2016

WORLD BREAKTHROUGH FOR WA RESEARCHERS

The international Duchenne muscular dystrophy (DMD) community is today rejoicing following the announcement by the U.S. Food and Drug Administration (FDA) it had approve a drug developed in Perth by WA researchers, which heralds a new frontier in the treatment of DMD.

DMD is a devastating and incurable muscle-wasting disease and one of the most common fatal genetic disorders, affecting one in every 3,500 males worldwide.

Close to two decades of research undertaken by a group led by Steve Wilton, Professor of Molecular Therapy at Murdoch University and Director of the Western Australian Neuroscience Research Institute (WANRI) and his colleague, Professor Sue Fletcher has culminated in the first ever treatment that is drastically improving the health and wellbeing of DMD suffers.

"Duchenne muscular dystrophy mainly affects boys, where every muscle in their body — including their heart — deteriorates, eventually leading to loss of life," said Professor Wilton.

"Until now, however, there has been no therapy that addresses the underlying genetic defect that causes DMD."

Clinical trials of the drug, eteplirsen, in the United States indicate that treatment reduces the severity of the disease by delaying the loss of muscle function. Boys, who have previously been told they are incurable, are still able to walk into their mid- to late-teens and potentially beyond.

"Through the trials we've seen the progression of these kids doing things they would not ordinarily being doing. Boys who would normally be in wheelchairs are instead running around playing football, jumping into cars," continued Professor Wilton.

"Having the drug approved by the FDA means more to Sue and I than any award we have ever received. Now for the first time our research will directly impact on the lives of young DMD sufferers worldwide."

Renowned neuroscientist and 2015 West Australian of the Year Professor Lyn Beazley AO applauded the research, saying it was "amongst the best medical research currently being undertaking in the world."

"Imagine if you could develop target-specific drugs and through a series of injections silence the faulty genes – a bit like putting white-out over a typographical error," Professor Beazley said.

Professor David Morrison, Murdoch University Deputy Vice Chancellor, Research and Innovation, praised the success of the researchers and the new drug.

"Murdoch University could not be prouder of Steve and Sue for their commitment and dedication to this ground-breaking research. It is not often researchers can take an idea from a concept through to market, but to see children benefiting from the product is extraordinary," Professor Morrison said.

"Murdoch University's translational research has been making a positive impact around the world for more than 40 years, and this breakthrough is further evidence of Murdoch's strong industry links making a difference to the world."

On the discovery, Steve Arnott, CEO of WANRI said:

"The game changing research of Professors Steve Wilton and Sue Fletcher and their team is a tribute to the world class researchers that have been associated with WANRI over decades.

Enhancing the lives of people through new discoveries such as these is a testament to what Western Australia can produce."

Eteplirsen works in about 1 in every 10 DMD patients, as DMD is caused by a variety of different mutations in the same gene, but Professors Wilton and Fletcher have developed other "genetic patches" that could be applied to other forms of DMD. Over time treatments should become available for the vast majority of DMD sufferers.

"This is really the thin end of the wedge for this type of therapy. Depending on the type of mutation, we can correct a variety of inherent diseases that have previously been considered untreatable," said Professor Wilton.

Eteplirsen is licensed to the US pharmaceutical company Sarepta Therapeutics to become available on prescription to DMD sufferers worldwide.

ENDS

NOTE TO EDITORS:

- Professors Wilton and Fletcher started their research into DMD at the University of Western Australia when they joined WANRI in 1991.
- Professor Wilton is the Director of WANRI.
- They joined Murdoch University and the Centre for Comparative Genomics in early 2013.
- Professors Wilton and Fletcher has received a number of accolades for their research in recent years. They are past winners of the WA Innovator of the Year award and received the 2013 Eureka Prize for Medical Research Translation – widely regarded as one of the "Oscars" of Australian science.

BACKGROUND

The Western Australian Neuroscience Research Institute (WANRI)

For over 30 years, the WA Neuroscience Research Institute (WA's oldest medical research institute) has provided residence to a world-class standard of medical researchers and specialists who have made breakthroughs across a broad spectrum of neurological disorders.

Sarepta Therapeutics

Sarepta Therapeutics is a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. Sarepta is also developing therapeutics for the treatment of rare, infectious and other diseases. For more information, please visit us at www.sarepta.com.

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