MEDIA RELEASE 19 SEPTEMBER 2016

Muscular Dystrophy WA is passionate about improving the quality of life of people living with muscular dystrophy. We believe that while we support those living with muscular dystrophy to live a life they love and reach their full potential, we are committed to ensuring we will never compromise the quest for effective treatment.

Today we bring you the exciting news that the U.S. Food and Drug Administration (The FDA) finally granted accelerated approval for Eteplirsen (Exondys 51) as a treatment for Duchenne muscular dystrophy. Many of you may be aware, this therapy has been a significant focus of the research undertaken by our Honorary Life Members Professors Steve Wilton and Sue Fletcher for the past 20 years.

While this is exciting step in the right direction for those with Duchenne muscular dystrophy who have a confirmed mutation of the dystrophin gene amenable to exon 51 skipping, it also provides significant hope for other DMD target exons and the development of future treatments across different genes and different conditions.

At this stage we cannot say when this drug will be available for our local community, but what we can say is that through the approval from the FDA, we know the drug is deemed to work and it is safe to use. From here Sarepta Therapeutics will conduct a two-year, randomised controlled trial from which the benefits of the drug will be assessed.

Muscular Dystrophy WA will continue to campaign for muscular dystrophy as a national health priority, we will continue to work collaboratively with our community, researchers, government and other industry organisations to ensure that through a national collaborative approach in the future we can create an environment where our WA community has direct access to treatments of this type.

Since our inception in 1967, our backbone of strength has been our commitment and investment into world renowned research. This investment has led to advances and support across many facets of our community. On behalf of the Members, Board and Staff at Muscular Dystrophy WA we wish to congratulate both Sue, Steve and respective teams at Murdoch University, WANRI and the University of WA (UWA) for their unfailing dedication and hard work to reach this stage.

Sue and Steve have thanked their research team, Muscular Dystrophy WA and especially the WA Duchenne families for their unwavering support over the previous 20 years.

WORLD BREAKTHROUGH FOR WA RESEARCHERS_RELEASE_2016

http://www.abc.net.au/news/2016-09-20/fda-approves-muscular-dystrophy-drug-eteplirsin/7861024

http://www.abc.net.au/news/2016-09-20/maddox-ball-has-a-form-of-duchenne-muscular-dystrophy/7861240

http://media.murdoch.edu.au/world-breakthrough-for-wa-researchers

http://www.prnewswire.com/news-releases/mda-celebrates-fda-accelerated-approval-of-eteplirsen-for-treatment-of-duchenne-muscular-dystrophy-300330196.html