Duchenne muscular dystrophy drug approved by FDA

21 September 2016, by Tony Malkovic, Sciencenetwork Wa

The powerful US Food and Drug Administration (FDA) has given the green light to a drug developed by WA researchers Sue Fletcher and Steve Wilton for treating Duchenne muscular dystrophy.

The Murdoch University scientists developed an innovative treatment to help sufferers of Duchenne muscular dystrophy, a crippling muscle-wasting disease that affects about one in 3 500 boys worldwide.

The FDA decision is a huge win for the global pharma company Sarepta Therapeutics, which has developed the drug under the name Eteplirsen.

In their breakthrough research, Professors Fletcher and Wilton had devised a way to bypass the faulty gene responsible for the disease, using a technique called exon skipping.

The FDA's approval follows an emotional campaign by sufferers, their families, and supporters of Eteplirsen.

Earlier this year, some 40 sufferers in wheelchairs and their families flew to Washington from around the US, and from as far as the UK, to show their faith in the treatment after authorities questioned aspects of the drug's clinical trial.

As reported in ScienceNetworkWA, Professor Fletcher's and Professor Wilton's innovative discovery had already won the 2012 WA Innovator of the Year Award.

In 2013, the researchers, then with UWA, signed a multi-million dollar deal with Sarepta to develop Eteplirsen.

Under the deal, they would get up to US$7.1 million in upfront and milestone payments, as well as royalties on the net sales of all medicines developed and approved.

This article first appeared on ScienceNetwork Western Australia a science news website based at Scitech.

Provided by Science Network WA