BioSpectrum the business of Bio & Health Sciences ASIA EDITION

FDA approves first new drug for ALS in 2 decades

11 May 2017 | News

The drug, known chemically as edaravone, has been sold by Japan-based Mitsubishi Tanabe in Japan and South Korea since 2015. The drug, known chemically as edaravone, has been sold by Japan-based Mitsubishi Tanabe in Japan and South Korea since 2015

After nearly two decades FDA has finally approved a drug for the treatment of amyotrophic lateral sclerosis (ALS). The drug, known chemically as edaravone, has been sold by Japan-based Mitsubishi Tanabe in Japan and South Korea since 2015.

ALS, or "Lou Gehrig's Disease," is a progressive neurodegenerative disease that affects nerve cells in the brain and the spinal cord. Motor neurons reach from the brain to the spinal cord and from the spinal cord to the muscles throughout the body. The FDA granted this drug orphan drug designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The new medicine, called Radicava is given intravenuously on a daily or near-daily basis for 14 days, followed by 14 days off the drug. A year's course of the medicine will cost about $145,000 before discounts to governments or insurance companies are included.

The new approval will bring much needed relief to millions of ALS patients across the world. Mr Eric Bastings, MD, deputy director of the Division of Neurology Products in the FDA's Center for Drug Evaluation and Research, “After learning about the use of edaravone to treat ALS in Japan, we rapidly engaged with the drug developer about filing a marketing application in the United States. This is the first new treatment approved by the FDA for ALS in many years, and we are pleased that people with ALS will now have an additional option."

The only other approved ALS medicine in the US is, generic riluzole, which modestly slows the progression of the disease in some people. As per reports, “the efficacy of edaravone for the treatment of ALS was demonstrated in a six-month clinical trial conducted in Japan. In the trial, 137 participants were randomized to receive edaravone or placebo. At Week 24, individuals receiving edaravone declined less on a clinical assessment of daily functioning compared to those receiving a

placebo.”

The data was presented last year at the AAN Annual Meeting, and showed the edaravone-treated group had a change in their ALS Functional Rating Scale-Revised (ALSFRS-R) score of -5.01 ± 0.64 compared to -7.50 ± 0.66 in the placebo group (*P* = .001). The most common adverse events were contusion (16%), and dysphagia (13%).

In 2014, ALS returned to the spotlight with the "Ice Bucket Challenge," which involved people pouring ice-cold water over their heads, posting a video on social media, and donating funds for research on the condition. The rare progressive condition attacks nerve cells located in the brain and spinal cord responsible for controlling voluntary muscles.

Eventually, the brain's ability to start and control voluntary movement is lost, and the patient succumbs to the disease - usually three to five years from the onset of symptoms.