FDA approves edaravone to treat MND

On 5 May, the US Food and Drugs Administration (FDA) approved the drug edaravone (which will be marketed as Radicava™) for the treatment of amyotrophic lateral sclerosis or motor neurone disease (MND) in the United States (US). It is the second MND treatment to be approved by the FDA; the first being riluzole that was approved in 1995.

We are currently liaising with our international colleagues and will keep the Australian MND community informed about further developments in the US and what they mean to people living with MND in Australia. This is what we know so far:

Background

The drug edaravone was developed by Mitsubishi Tanabe (MT) Pharma Corporation in Japan. Edaravone was originally marketed for use in stroke patients. Later, the company decided to test edaravone in people with ALS/MND. After a series of Phase 3 trials in Japan in 2015, regulatory bodies approved edaravone to treat people with ALS/MND in Japan and South Korea. Edaravone is marketed under the brand name Radicut® in Japan and South Korea.

In June of 2016, MT Pharma America submitted an application to the FDA for regulatory approval in the US. This application was approved by the FDA on 5 May 2017. Edaravone will be marketed as Radicava™ in the US and will be distributed commercially by MT Pharma America.

What is edaravone?

Edaravone is a drug with antioxidant properties. It protects nerve cells by mopping up damaging “free radicals” in the body.

Clinical trials have shown edaravone slows the progression of MND, potentially helping people preserve function longer. It appears to work in a subset of people and is most beneficial as an early treatment. The complete results of the final study have not yet been published in a scientific journal.

Edaravone is administered at a hospital or clinic by a doctor via an intravenous infusion. It should be administered in 28-day cycles. For the initial cycle, the treatment is infused for 14 consecutive days, followed by a two-week drug-free period. All cycles after that are infused for 10 days within a 14-day period, followed by a two-week drug-free period. It takes 60 minutes to

receive each 60 mg dose.

A company called Treeway is currently developing an oral preparation of the drug for testing.

Some adverse reactions of edaravone have been reported and include bruising, walking difficulties, headache, inflammation, eczema and dermatitis.

Access to edaravone

At this time, edaravone is only approved for use in Japan, South Korea and the United States. Based on current information, it is anticipated edaravone will be available in the US from August 2017.

The Therapeutic Goods Administration (TGA) is yet to approve edaravone to treat MND in Australia.

MND Australia's position statement on the Development and approval of drugs to treat MND outlines ways that patients can gain access to drugs that have not been approved for use in Australia and includes the Special Access Scheme. It should be noted this process can be challenging and expensive for the person living with MND and their family. The estimated annual cost of edaravone is US$146K.

People with ALS/MND have attempted to access edaravone in Japan. A representative of the Japan ALS Association has provided a FAQ document that explains how international patients may access Radicut® in Japan. This process can be challenging and expensive. It also requires the cooperation of a local medical professional to administer the drug.

Working towards making edaravone available in Australia

As the national advocate representing all Australians who are impacted by MND, MND Australia aims to harness the heightened awareness of MND and collective voice of the MND community to put edaravone on the radar of key decision makers with a call to accelerate TGA approval for use in Australia.

MND Australia and the State MND Associations spearheaded the lobbying efforts for approval of riluzole by the TGA in 2001 and subsequent listing on the Pharmaceutical Benefits Scheme in 2003. We will continue to work fervently to ensure that safe, effective drugs such as edaravone are made available to people living with MND in Australia.

News from Members of the International Alliance of ALS/MND Associations

The ALS Association: Press Release
The ALS Association: FAQ About Radicava
ALS Therapy Development Institute

ALS Hope Foundation
Les Turner ALS Foundation
MND Association of England, Wales and Northern Island

Other Resources
Official FDA Announcement
Official MT Pharma America Press Release
Reuters News Story