A second ALS treatment, edaravone, has been newly approved in the United States

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Highlights:

- The United States Food and Drug Administration recently approved edaravone (also referred to as Radicava and Radicut) for the treatment of ALS. It's only the second ALS drug to be approved in the US. The first drug, riluzole, was approved more than 20 years ago. The ALS Association says the drug will be available in the US in August 2017.

- Radicava is delivered intravenously through infusions over a number of days, with 14 day breaks in between.
- Clinical trial data from the manufacturer, Mitsubishi Tanabe, suggest that Radicava can be effective in improving the condition of people who are early in their ALS progression. A New York Times article describes the drug's effect as "modest but significant."
- Radicava may help only a certain segment of people living with ALS, but it is encouraging to see the introduction of a second treatment option in the U.S.
- Now that Radicava has been approved by the FDA, we are hopeful that Mitsubishi Tanabe will take steps for Canadians to be able to access the drug here.
- The hope is that the FDA's approval of Radicava will help to build further momentum for drug manufacturers to focus on ALS for the development of other therapies, underscoring the need for continued research investment to drive additional scientific discovery.

Background

Edaravone is the generic name for a drug that is marketed as Radicava and sometimes Radicut. Manufactured by the Mitsubishi Tanabe Pharma Corporation, it has been used in Japan since 2001 as a treatment for stroke. Clinical trials testing Radicava as a treatment for ALS have been taking place in Japan since 2006 and in recent years the drug was approved in Japan and South Korea for treating ALS. On May 5, 2017 the U.S. Food and Drug Administration **approved Radicava** as a treatment for ALS – the first in more than 20 years, and only the second ALS drug to be approved in the US. According to the U.S.-based **ALS Association**, Radicava will be available in the U.S. in August 2017 and the **New York Times** reports it will cost $145,254 USD per year.

Who can benefit from treatment with Radicava?

In an earlier clinical trial, Radicava was tested in people in varying stages of ALS progression, and the drug did not show significant slowing of the disease. However, a **more recent clinical trial** recruited participants who were early in their ALS progression, with milder symptoms and a larger vital capacity (the maximum amount of air a person can expel from their lungs after a maximum inhalation). There were 137 participants in this trial, some of whom received the drug and some of whom received a placebo. According to Mitsubishi Tanabe, after six months the condition of the participants who received the drug declined less than the patients receiving the placebo, as measured using the **ALS Functional Rating Scale**. This result is described in the New York Times article as "modest but significant." Results from the most recent clinical trial have not yet been published in a peer-reviewed journal, which in the scientific community is an important indication of the validity of the data. According to the New York Times, there is no information available as to whether Radicava could help people to live longer with ALS.

How is Radicava administered?

Treatment is delivered intravenously every day for 14 straight days, followed by no drug for the next 14 days. This is followed by intravenous treatment over 10 of the next 14 days followed by another 14-day period without the drug. This continues for as long as the treatment is to be administered.

What's next?

Radicava may help only a certain segment of people living with ALS, but it is encouraging to see the introduction of a second treatment option in the U.S. Now that Radicava has been approved by the FDA, we are hopeful that Mitsubishi

Tanabe will take steps for Canadians to be able to access the drug here. Should Radicava be made available in Canada, the costs may be different than in the U.S.

As has been seen with cancer and HIV/AIDS, the introduction of new therapies can help to create momentum for drug manufacturers to pursue the development of additional treatment options. We are hopeful the FDA's approval of Radicava will have a similar impact on the development of ALS therapies – which also underscores the need for continued research investment so the scientific community can continue to pursue discoveries. This in turn will inform the development of additional therapeutic options with even greater potential to make ALS a treatable, not terminal disease.