



EMORY ALS CENTER

Celebrate Life...Imagine a cure

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Dear ALS patients:

We are writing this letter to alert you to the recent approval of Radicava (generic name edaravone) for the treatment of ALS. While having the first new drug approved for ALS in more than 20 years is certainly an exciting development, we think it is important to understand the data that led to the approval of the drug and to discuss the logistics of how the drug is administered. The preliminary study of the drug looked promising, but a confirmatory study, performed on a larger number of unselected patients with ALS, was negative. When subgroups of patients in the confirmatory study were analyzed, it was noted only selected patients benefited. These patients had neither rapid nor slow progression, were recently diagnosed, and had normal respiratory function. In the study that led to approval by the FDA, these were the only patients included. The study that led to approval demonstrated a slowing of progression on the rating scale known as the ALSFRS-R. This is a numerical scale that measures physical function of ALS patients. The effect of the drug on life expectancy was not analyzed (in the prior confirmatory study there was no benefit). The FDA's approval of Radicava for ALS does not specify any subgroup of patients. Whether the drug will slow progression in the patients that were excluded from this study is unclear, but the prior confirmatory study did not show slowing of progression in a group of unselected ALS patients.

Radicava is administered by an intravenous infusion. It will be given for 14 days consecutively with a 14 day rest for the first cycle followed by 10 days out of every 28 days thereafter. This means that in the maintenance phase, patients will be receiving an IV infusion 130 times per year. This frequent IV infusion is likely to require the surgical placement of a device known as a port, which allows for frequent intravenous access. Ports are often used for administering chemotherapy. The drug will likely be given at home by an infusion nurse or at an infusion center. The infusion (not including set up before and clean up after) will take 1 hour.

Radicava is estimated to cost about \$146,000 per year, which does not include other related costs. These other costs include surgical placement of the port and service charges from infusion centers and home infusion companies. At this point, we do not know what criteria insurance companies will use to decide whether or not to cover the cost of the medication. We also do not know what the out of pocket cost will be. The manufacturer of the drug, Mitusubishi Tanabe Pharma American (MT Pharma), is going to offer free case management services to help patients secure coverage through their health insurance company. MT Pharma states that it will provide a financial assistance program to help with copayments. Additionally, if you do not have health insurance and meet certain income and other requirements, they will provide Radicava at no cost. For more information on these programs, please contact MT-Pharma at 844-772-4548 or visit their website www.mt-pharma-america.com.

The drug is expected to be available for patients in the US beginning in August, 2017. We encourage patients and caregivers to familiarize themselves with the information available and be prepared to discuss the drug with your physician. For more information, we recommend visiting the ALS Association's website, which has some educational material regarding Radicava (www.alsa.org/research/radicava/).