Real-World Survival in Edaravone-Treated ALS

BACKGROUND
Amyotrophic lateral sclerosis (ALS) is a fatal neurodegenerative disease characterized by progressive paralysis, requiring death or assisted ventilation in the majority of patients. In short-term randomized trials of IV edaravone, no statistically significant effect of IV edaravone on survival (HR, 0.65; 95% CI, 0.59-0.91) compared with the non-IV edaravone–treated controls (n = 318; n = 208; 65.4% vs 18.6%) was observed. 

OBJECTIVE
To evaluate overall survival in patients with ALS receiving IV edaravone from claims submitted for all medical and pharmacy healthcare services from January 2007 through December 2020. The US Food and Drug Administration (FDA) approved edaravone for the treatment of ALS in May 2017 and May 2022, respectively.

METHODS

Patient selection. Data were collected from patients with ALS before the index date (preindex data) and who had commercially available coverage (commercial vs Medicare Advantage) from January 2005 through March 2020. Adults ≥18 years of age on the index date were included in the study if they initiated IV edaravone between August 8, 2017, and March 31, 2020, with a control intervention (non-IV edaravone–treated patients based on the nearest-propensity score match). Examined the assumption of noninformative censoring by nonrandomized, interventional studies by adjusting for known confounding factors in a propensity score model using the nearest-neighbor method to achieve a mixed population of treated or untreated patients with varying characteristics to achieve a nonrandomized, interventional study setting.

Statistical analysis. All-cause survival endpoints were analyzed using the Kaplan-Meier method to compare survival between treatment groups and to estimate the proportion of patients alive at 30 months. For the survival analysis, patients with ALS were censored at the date of the last claim or end of the study period (March 31, 2021). The Wilcoxon rank-sum test for continuous variables and the chi-square test for categorical variables were used to compare covariates. Univariate analysis was used to test categorical and clinical characteristics between the 2 groups. Survival analysis was performed using the Kaplan-Meier method to compare survival between treatment groups.

RESULTS

The study population included 184 patients with ALS who were alive at 12 months and 18 patients who were alive at 24 months. The survival rate at 30 months among IV edaravone–treated patients was 20.0% (n = 31; 95% CI, 13.0-27.0) compared with the non-IV edaravone–treated controls (n = 31; 95% CI, 20.0-28.0).

CONCLUSIONS
The results of this analysis suggest that IV edaravone has no statistically significant effect on overall survival compared with the non-IV edaravone–treated controls. This analysis was exploratory, and more randomized clinical trials are needed to determine the role of IV edaravone in improving survival in patients with ALS.

LIMITATIONS
This study was limited to patients with ALS who had commercial health insurance. The study relied on administrative claims data, which are subject to biases due to coding variances, underreporting, and nonreporting. The study results should be interpreted within the context of the specific healthcare population (commercial or Medicare Advantage) included in this study.

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REFERENCES