Real-World Treatment Patterns in Amyotrophic Lateral Sclerosis Patients Treated With IV Edaravone: 12-Months of Follow-up in Patients Receiving Treatment From a National Infusion Center

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BACKGROUND

- Intravenous (IV) edaravone was approved by the United States (US) Food and Drug Administration (FDA) for the treatment of amyotrophic lateral sclerosis (ALS) in May 2017 and became available to US health care providers in August 2017
- The approval was based upon a pivotal, randomized, controlled trial in which IV edaravone was shown to slow down the loss of functional decline on the ALS Functional Rating Scale-Revised (ALSFRS-R) by 33% (P=0.0013) compared with placebo at 24 weeks[1]
- IV edaravone is administered by infusion at clinic sites, infusion centers, or at home[2,3]
- As 1 of only 2 drug therapies approved for the treatment of ALS in the US, there is interest in the real-world experience with IV edaravone
- However, to date, real-world evidence (RWE) on the use of IV edaravone in the US has been limited

HYPOTHESIS

- Patient data collected by a provider of home and alternative-site infusions may help elucidate real-world treatment patterns with IV edaravone in the US

METHODS

- A retrospective cohort study was conducted using the Soleo Health de-identifiable database
- Soleo Health is a provider of home and alternative-site infusions and specialty pharmacy services in the US and has collected data on ALS patients treated with IV edaravone since the time of commercial availability
- Soleo Health nurses and other allied health care professionals collected the patient data during home visits for administration of infusions, or in some cases by phone for those patients who perform their own infusions
- ALS Patients included in this analysis were treated with IV edaravone for at least 3 consecutive months between August 8, 2017 and March 31, 2020
- Variables collected and described in this analysis include:
  - Patient demographics
  - Disease characteristics
  - ALS Functional Rating Scale-Revised (ALSFRS-R) scores
  - Treatment outcomes

Statistical Analysis

- Demographics and clinical variables were assessed descriptively using counts and percentages for categorical variables and measures of central tendency (mean/median/standard deviation/interquartile range, IQR) for continuous variables

RESULTS

Patient Characteristics and Treatment Outcomes

- The US Distribution of ALS patients in the Soleo database receiving IV edaravone are shown in **Figure 1**
- A total of 167 patients were included in the analysis. Mean age±SD was 60.4±11.3 years, 53.3% were male, mean baseline ALSFRS-R score was 37.1±9.24, and 60.6% of patients were covered by commercial plans. **Table 1** shows the demographic characteristics of ALS patients by start date of IV edaravone. In the total sample the mean±SD ALSFRS-R at baseline was 36.9±9.1

Figure 1. US distribution of ALS patients in the Soleo database receiving IV edaravone

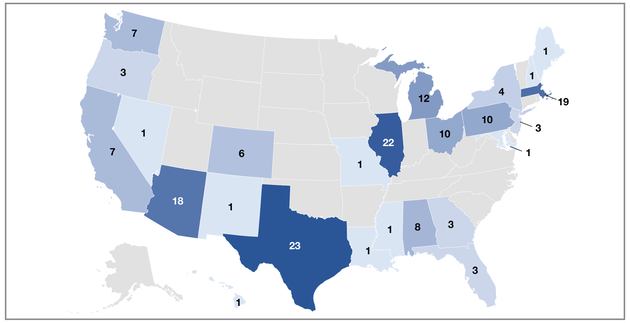


Table 1. Patient counts, demographic characteristics and ALSFRS-R* score by year of IV edaravone initiation

	2017	2018	2019	Total
N	51	103	13	167
Gender, n (%)				
Male	30 (58.8)	55 (53.4)	4 (30.8)	89 (53.3)
Female	21 (41.2)	48 (46.6)	9 (69.2)	78 (46.7)
Age group, n (%) years				
18 – 39	3 (5.9)	5 (4.9)	0	8 (4.8)
40 – 49	6 (11.8)	12 (11.7)	0	18 (10.8)
50 – 59	17 (33.3)	28 (27.2)	8 (61.5)	53 (31.7)
60 – 69	14 (27.5)	32 (31.1)	1 (7.7)	47 (28.1)
70 – 79	9 (17.6)	23 (22.3)	4 (30.8)	36 (21.6)
80+	2 (3.9)	3 (2.9)	0	5 (3.0)
Age (mean, SD) years	59.1 (11.6)	60.7 (11.5)	62.4 (9.1)	60.4 (11.3)
Age (median, range) years	58.0 [51.0, 67.0]	62.0 [52.5, 69.5]	58.0 [58.0, 70.0]	60 [33 – 84]
ALSFRS-R at baseline, mean (SD)	33.5 (6.5)	37.1 (9.6)	38.1 (7.5)	36.9 (9.1)
Commercial Plan, n (%)	27 (52.9)	66 (64.1)	8 (61.5)	101 (60.5)
Managed Medicare, n (%)	24 (47.1)	36 (35.0)	5 (38.5)	65 (38.9)
Missing insurance information, n (%)	0	1 (0.9)	0	1 (0.6%)

*ALS Functional Rating Scale-Revised

- During the 12-month follow-up period, 89 (53.3%) patients discontinued treatment. The most frequent reason for discontinuation was patient choice, 34 (38.2%), followed by death or hospice, 23 (25.8%), and insurance or care change, 20 (22.5%) (**Table 2**)

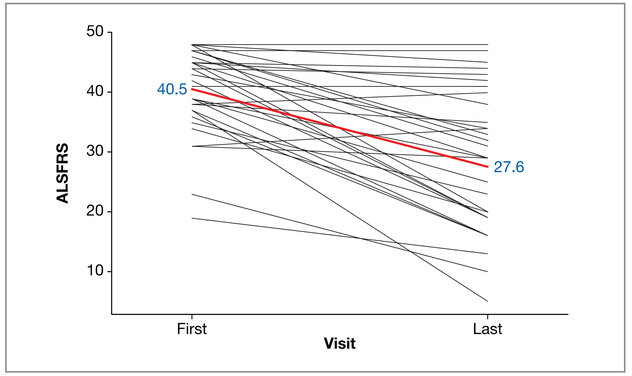
Table 2. Treatment outcomes

	Start of IV edaravone treatment			Overall
	2017	2018	2019	
N	49	102	16	167
Continuing Treatment, n (%)	20	46	12	78 (46.7)
Discontinued Treatment, n (%)	29	56	4	89 (53.3)
Discontinue Reason, n (%)				
Death or Hospice	7 (24.1)	13 (23.2)	3 (75.0)	23 (25.8)
Insurance or Care Change	8 (27.6)	12 (21.4)	0 (0.0)	20 (22.5)
Other	5 (17.2)	7 (12.5)	0 (0.0)	12 (13.5)
Patient Choice	9 (31.0)	24 (42.9)	1 (25.0)	34 (38.2)
ALSFRS-R at end of treatment, mean (SD)	27 (11.3)	29 (11.0)	30.2 (7.4)	28.4 (10.9)
Treatment duration (month), median (IQR)	17.0 [11.2, 21.6]	11.0 [6.6, 14.6]	5.6 [4.3, 6.4]	11.8 [6.6, 16.4]

ALSFRS-R Score Data

- In the 78 patients who continued treatment and had repeat ALSFRS-R scores, the mean±SD ALSFRS-R score at the start of treatment was 40.5±7.2, and at the last treatment visit, 27.6±11.8 with a mean change per month of –0.4±0.52. The median treatment duration was 357 days (**Figure 2**)

Figure 2. ALSFRS-R score changes in patients who continued treatment (n=78)



Red line indicates the change in mean ALSFRS-R score from the first visit in patients who continued treatment to the mean ALSFRS-R score at last visit

ALSFRS scores: Baseline: 40.5±7.2; Last: 27.6±11.8; Mean change per month = –0.4; SD Change = 0.52

LIMITATIONS

- This study is limited to ALS patients who receive treatment through infusion services; therefore, the results may not be generalizable to all ALS patients
- The study relies on electronic health record data and self-reported data, which may be subject to coding and entry error and bias
- Pre-observational ALSFRS-R scores were not included

CONCLUSIONS

- This analysis describes treatment persistence for IV edaravone in a real-world setting as reported by Soleo Health
- The changes in ALSFRS-R score reported were consistent with changes observed in the pivotal Phase 3 study, Study 19, which showed a 33% slowing of the rate of functional decline with edaravone treatment and was the basis for IV edaravone's FDA approval[1]
- It is hoped that this information will be useful to clinicians who prescribe IV edaravone for their patients with ALS

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ACKNOWLEDGMENTS AND DISCLOSURES

Acknowledgments

p-value communications provided editorial support.

Disclosures

MH, AH, MC, and SA are employees of Mitsubishi Tanabe Pharma America (MTPA).
BP is an employee of Soleo Health.
YL and JZ are employees of Princeton Pharmatech.

The study was funded by MTPA.

MA-RC-US-0159

Presented at the Northeast Amyotrophic Lateral Sclerosis (NEALS) Consortium Annual Meeting | September 30 - October 1, 2020