Treatment Persistence Among Amyotrophic Lateral Sclerosis Patients Receiving Intravenous Edaravone: Results From a National Infusion Center

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BACKGROUND

- Intravenous (IV) edaravone (Radicava®; Mitsubishi Tanabe Pharma Corporation, Tokyo, Japan) 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
- To date, information on the real-world effectiveness in patients treated with IV edaravone in the US is limited

OBJECTIVE

- To describe real-world treatment patterns in patients treated with IV edaravone collected by a provider of home and alternative-site infusions

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 in 2017
- 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
  - ALSFRS-R scores
  - Treatment outcomes
  - Patient reported questions addressing energy levels, stress levels, and overall health and wellness
- Wellness information was collected by Soleo Health nurses and other allied health care professionals at each visit with a defined questionnaire (**Table 1**)
- The questionnaire consists of 5 questions, each with a set of 5 possible answers recorded on a scale of 1 to 5
- Larger scale numbers indicate a deterioration of wellness

Table 1. Wellness questions

Category	Question	Answers
Energy level	How would you describe your energy level and ability to participate in activities?	1. Lots of energy and can do things I want to do 2. Can do most things, may need to rest at times 3. Normal level of energy 4. Feel tired most of the time, spend a lot of time sitting and not engaging in activities 5. Staying in bed most of the day due to fatigue, tired all day
Stress level	How would you describe your stress level?	1. Very relaxed, feel at peace 2. Relaxed 3. Normal stress level and feeling calm 4. Somewhat stressed 5. Very stressed
Overall health and wellness	On a scale how would you describe your feeling of health and wellness?	1. Feel great 2. Feel good 3. Feel okay 4. Feel bad 5. Feel bad all the time

Statistical Analysis

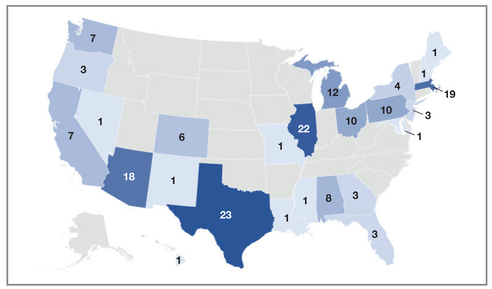
- Demographics and clinical variables were assessed descriptively using counts and percentages for categorical variables and measures of central tendency (mean/median/standard deviation/interquartile range, IQR) for continuous variables

RESULTS

Patient Characteristics and Treatment Outcomes

- The US Distribution of ALS patients in the Soleo database receiving IV edaravone are shown in **Figure 1**

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



- A total of 167 patients were included in the analysis
- Mean age±SD was 60.4±11.3 years, 53.3% were male, and 60.6% of patients were covered by commercial plans (**Table 2**)
- In the total sample the mean ±SD ALSFRS-R at baseline was 36.9±9.1

Table 2. 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%)

- 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 3**)

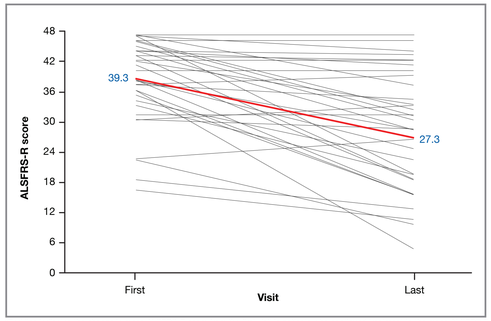
Table 3. Treatment outcomes

	Start of IV edaravone treatment			
	2017	2018	2019	Overall
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 (46.7% of the total sample) patients who continued treatment and had repeat ALSFRS-R scores, the mean±SD ALSFRS-R score at the start of treatment was 39.3±7.9, and at the last treatment visit, 27.3±11.2 with a mean change per month of –0.46±0.66 at month 12 (**Figure 2**)
- The median treatment duration was 19.7 months (13.1, 25.4)

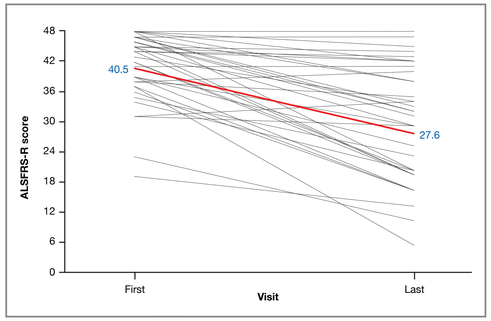
Figure 2. ALSFRS-R score changes in patients who continued treatment (n=78)[a]



[a]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

- At the end of the study period, 64 (38.3% of the total sample) patients had more than 12 months of continuous treatment with IV edaravone
- In these patients, 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 (**Figure 3**)
- The median treatment duration was 22.6 months (range 12.2 to 30.8 months)

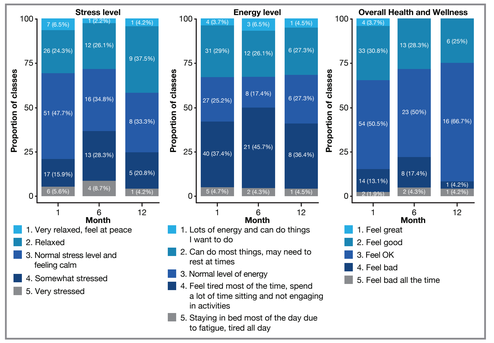
Figure 3. ALSFRS-R score changes in patients who had greater than 12 months continuous treatment with IV edaravone (n=64)[a]



[a]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

- At the end of the 12 month of IV edaravone treatment, 33.3% of patients reported having normal stress levels and feeling calm; 36.4% reported feeling tired most of the time; however, 25% reported feeling good in their overall health and wellness and 66.7% of patients reported feeling okay (**Figure 4**)

Figure 4. Patient reported stress level, energy level, and overall health and wellness scores collected at month 1, 6, and 12 of IV edaravone treatment



LIMITATIONS

- This study was limited to ALS patients who received treatment through infusion services; therefore, the results may not be generalizable to all ALS patients
- The study relied 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 and patient reported wellness questions were not collected by a validated questionnaire

CONCLUSIONS

- This analysis describes treatment persistence and self-reported health and wellness scores in patients treated with 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 IV 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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Disclosures
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