Real-World Evidence of Radicava® (edaravone) for Amyotrophic Lateral Sclerosis From a National Infusion Center Database in the United States

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BACKGROUND

- Radicava® (edaravone) was approved by the United States (US) Food and Drug Administration for the treatment of amyotrophic lateral sclerosis (ALS) in May 2017 and became available to US health care providers in August 2017
- A pivotal, randomized, controlled clinical study conducted in Japan showed that edaravone slowed the rate of functional loss in ALS[1]
- Radicava® 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, and because the pivotal clinical studies for edaravone were conducted in Japan, there is interest in the real-world experience with Radicava®. However, to date, real-world evidence (RWE) on the use of Radicava® in the US has been lacking

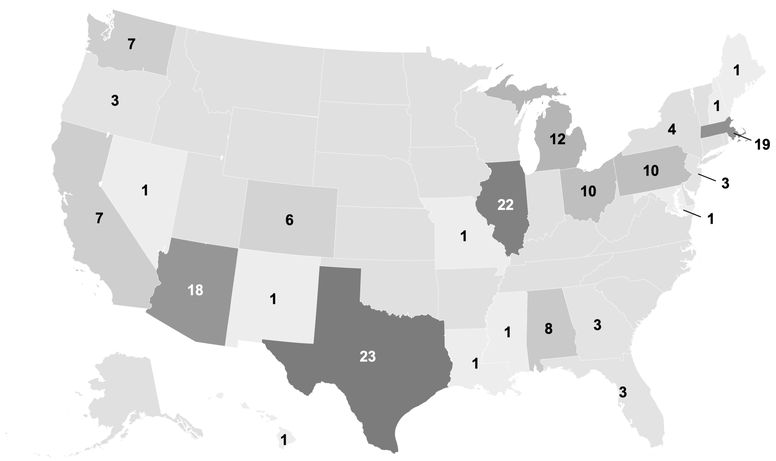
OBJECTIVE

- To provide RWE of Radicava® use from data collected by a provider of home and alternative-site infusions

METHODS

- Soleo Health, a provider of home and alternative-site infusions and specialty pharmacy services in the US, collected a database of RWE on the use of Radicava® since the time of commercial availability in August 2017 through August 2019
- Patients in the database (N=167) had been receiving Radicava® for ALS for at least 3 months; their distribution is shown in **Figure 1**

Figure 1. US Distribution of Soleo ALS Patients Receiving Radicava®



- A retrospective analysis of this database was conducted
- Analysis parameters included the following:
  - Patient demographics
  - Disease characteristics
  - ALS Functional Rating Scale-Revised (ALSFRS-R) scores
  - Quality-of-life and wellness information
  - Reports of adverse drug reactions
- The patient data were collected by Soleo Health nurses and other allied health care professionals during home visits for administration of infusions, or in some cases by phone for those patients who perform their own infusions
  - ALSFRS-R data were collected using the standardised set of questions for the scale
  - Wellness data were collected with a set of 5 questions, each with a scale of potential answers (larger scale numbers indicate a deterioration of wellness) (**Table 1**)

Table 1. Wellness questions

Category	Question	Answers
Sleep pattern	How would you describe your sleep patterns?	1, Great restful sleep; 2, Good sleep; 3, No real problem; 4, Have problems falling asleep or staying asleep; 5, Insomnia
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
Health	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
Mood	How would you describe your mood?	1, In a great place and feel positive; 2, Good mood most of the time; 3, Feel even tempered; 4, Feel blah some of the time, sometimes feel cranky; 5, Depressed all of the time, irritable and annoyed with others
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

- Usage patterns for Radicava® were also analysed

RESULTS

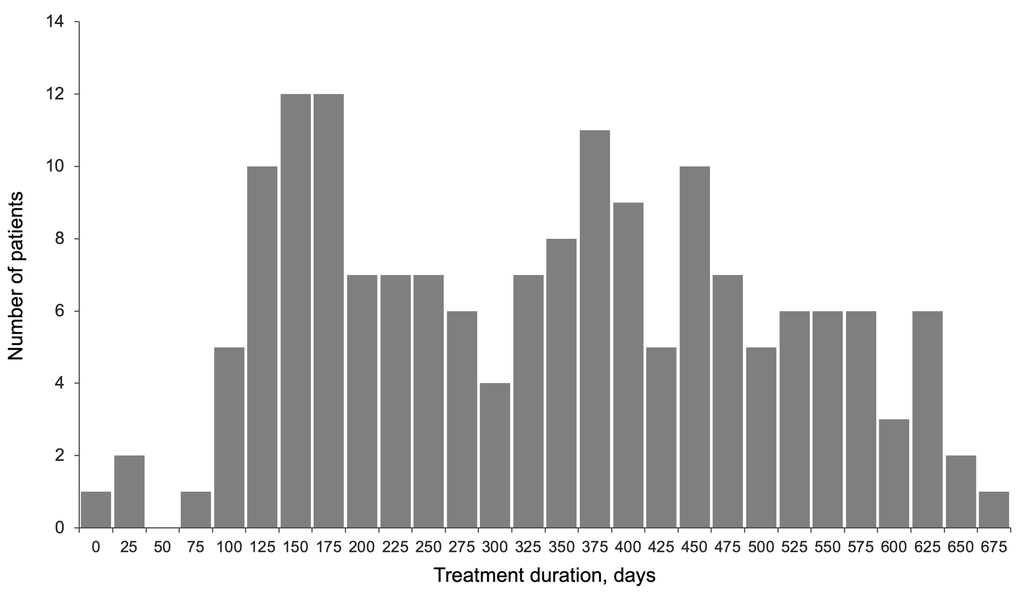
- Characteristics of the patients in the database are shown in **Table 2**

Table 2. Patient characteristics

Characteristic	Patients (N=167)
Gender, n (%)	
Female	78 (46.7)
Male	89 (53.3)
Age, yr, mean ± SD	60.4 ± 11.3
Allergy to any drug, n (%)	
No	110 (65.9)
Yes	57 (34.1)

- The patients had been using Radicava® for a median of 331.5 days (range, 0–663 days) per patient (**Figure 2**)

Figure 2. Radicava® treatment duration



- ALSFRS-R scores are shown in **Table 3**

Table 3. ALSFRS-R scores (n=75)

Measurement timing	Mean ± SD
Month 1[a]	37.08 ± 9.27
Month 6	34.16 ± 9.98
Change from month 1 to 6 (units/month)	−0.62 ± 0.97

[a]The month 1 measurements occurred within 30 days of starting on therapy.

- Patient wellness data are shown in **Table 4**

Table 4. Wellness scores

		Score (mean ± SD)		
Variable	Patients (n)	Month 1[a]	Month 6[b]	Change[c]
Sleep pattern	108	2.45 ± 1.16	2.51 ± 1.13	001 ± 1.05
Stress level	103	2.89 ± 1.01	2.85 ± 1.05	−0.05 ± 0.93
Health	105	2.68 ± 0.86	2.63 ± 0.86	−0.05 ± 0.92
Mood	104	2.37 ± 1.00	2.42 ± 1.02	0.08 ± 0.97
Energy level	103	2.78 ± 1.00	2.77 ± 1.03	0.01 ± 0.86

[a]The month 1 measurements occurred within 30 days of starting on therapy.
[b]Last observation carried forward for patients with a last visit between 3 and 6 months.
[c]For patients with both month 1 and month 6 data.

- Adverse events reported during the analysis period are listed in **Table 5** and the serious adverse events are listed in **Table 6**

Table 5. Adverse events reported (n=34/167 patients)

Adverse effect	Number of reports
Total AEs	**103**
Drug ineffective	11
Fall	8
Asthenia	7
Muscular weakness	7
Disease progression	6
Condition aggravated	4
Death	3
Fatigue	3
Gait disturbance	3
Therapeutic response unexpected	3

Table 6. Serious adverse events reported (n=9/167 patients)

Adverse effect	Number of reports
Total SAEs	**15**
Death	3
Acute kidney injury	1
Back pain	1
Bacteraemia	1
Blood potassium increased	1
Catheter site infection	1
Choking	1
Deep vein thrombosis	1
Fall	1
Heart rate abnormal	1
Joint injury	1
Muscular weakness	1
Pneumonia	1

DISCUSSION AND CONCLUSIONS

- This report provides RWE on the use of Radicava® in the US from data collected by an infusion and specialty pharmacy services provider
- Among the 167 patients providing data for analysis, the median duration of Radicava® therapy was 331.5 days
  - Changes in ALSFRS-R score during the analysis period averaged approximately –0.62 ± 0.97 units per month
  - Wellness data indicated minimal changes during the analysis period
  - In this report, no unexpected safety signals were seen, nor any inconsistencies with the clinical trials
    - The limitations stemming from voluntary reporting and occasional missing information should be considered when interpreting these results
- Limitations of this study include the lack of an edaravone-naïve comparison population, pre-observational ALSFRS-R score data, and ALSFRS-R score data collected by allied health care professionals in order to replicate inter-observer reliability
- The data are expected to be helpful for clinicians who are considering using Radicava® therapy with their ALS patients

REFERENCES

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Disclosures

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