Optimizing Access to Care in ALS

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Introduction

ALS is a is a progressive, currently incurable neurodegenerative disorder that causes muscle weakness, disability, and eventually death. ALS is a rare disease, affecting nearly 3 to 7 people in 100,000 per year in Europe and North America.1 ALS mainly affects the elderly, though it can affect adults at any age (Figure 1). Most ALS patients die within three to five years of diagnosis; 30% of ALS patients are alive five years after diagnosis, and 10%-20% survive for more than 10 years.[2]

While there is currently no cure, a well-orchestrated, multidisciplinary treatment plan can improve quality of life and increase survival. The integrated multidisciplinary team will include a neurologist, pulmonologist, speech therapist, assistive technology specialist, physical and occupational therapists, and psychosocial support.[3]

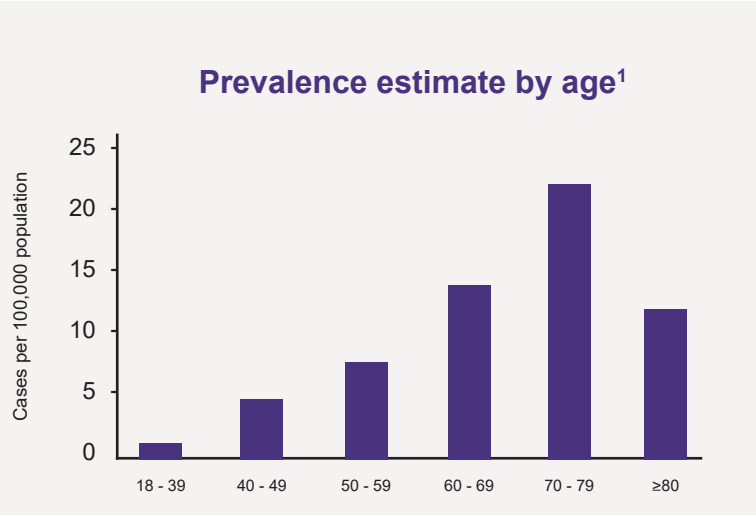

Figure 1: While ALS prevalence is highest among individuals older than 60, it can affect adults at any age[1]

Optimal care for ALS can be hindered by structural, financial, and institutional barriers. Many clinicians do not have direct experience treating patients with ALS and may not be aware of available resources or evidence-based supportive therapy. Experienced ALS centers have developed strategies to overcome barriers to ensure access to treatments and equipment. This includes philanthropic resources, strategic utilization of insurance benefits, implementation of patient-centered evidence-based care, and appropriate clinical documentation that ensures insurance reimbursement.

The cases presented in this document illustrate three key phases of the ALS patient journey and the clinical decisions at each phase.

- **Case 1: Newly Diagnosed**
- Case 2: Respiratory Presentation with Bulbar and Upper Extremity Weakness
- Case 3: Respiratory Disease with End-of-Life Care

Clinical decisions in the initial phases of the disease are characterized by ensuring access to clinical trials, disability, physical therapy, and available medication. Thorough documentation of disease diagnosis and progression is essential for coverage. Genetic testing is recommended at this stage, as it can affect eligibility for clinical trials and potential new therapies in the future.

1. Mehta P, Kaye W, Raymond J, et al. Prevalence of Amyotrophic Lateral Sclerosis - United States, 2014. MMWR Morb Mortal Wkly Rep. 2018;67(7):216-218.
2. Kiernan MC, Vucic S, Cheah BC, et al. Amyotrophic lateral sclerosis. Lancet. 2011;377(9769):942-955.
3. Traynor BJ, Alexander M, Corr B, Frost E, Hardiman O. Effect of a multidisciplinary amyotrophic lateral sclerosis (ALS) clinic on ALS survival: A population based study, 1996-2000. J Neurol Neurosurg Psychiatry. 2003;74(9):1258-1261.

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Patient #1: Newly Diagnosed

52-year-old white female

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Symptom Presentation & Initial Care Management

- 9 months prior to diagnosis - right distal leg weakness, drift to the right when walking, fall and fracture distal fibula on left leg
- 7.5 months prior to diagnosis - weakness in right hand, able to raise arms above head
- Progression of bilateral leg and hand weakness
- Confirmed diagnosis, C9orf72 negative
- Started riluzole with follow up AST/ALT blood monitoring 30/60/90 days after first dose

Clinical Considerations and commentary

- Diagnosis is often delayed by an average of 12 months after symptom onset (Figure 2)[5,6]
- Genetic testing can influence future clinical decisions: The drug development pipeline includes several agents targeted at a specific genetic defect, and eligibility to those trials are determined by a genetic diagnosis. The most common ALS-associated genes are C9orf72, SOD1, FUS, and TARDBP. Historically, genetic testing was offered only to those who were suspected of having familial ALS, but increasingly it is recommended that all patients with ALS undergo a genetic test[7]
- In addition to a genetic diagnosis, eligibility for clinical trials is often restricted by time from symptom onset[8]

Initial ALS Clinic Visit

Discussed:

- Disease confirmation with patient and family members — reviewed testing
- Recommend local ALS multidisciplinary clinic
- Current clinical drug trials and ongoing ALS research
- ALS treatment medications — riluzole and Radicava — actions, side effects, cost, treatment expectations, required insurance pre-authorizations
- Triaged current limitations — recommend local PT/OT evaluation
- Sick time, medical leave, short & long term disability; applying for Social Security disability
- Consideration of renovations to home
- Family support
- Adaptive devices for ADLs
- Safe exercise program including aqua therapy supervised by physical therapist experienced in ALS[1]
- Ongoing clinical evaluation for nutritional[2] & respiratory support therapy in near futureEMST (expiratory muscle strength training)[3] and voice/ message banking[4]
- Advance directives

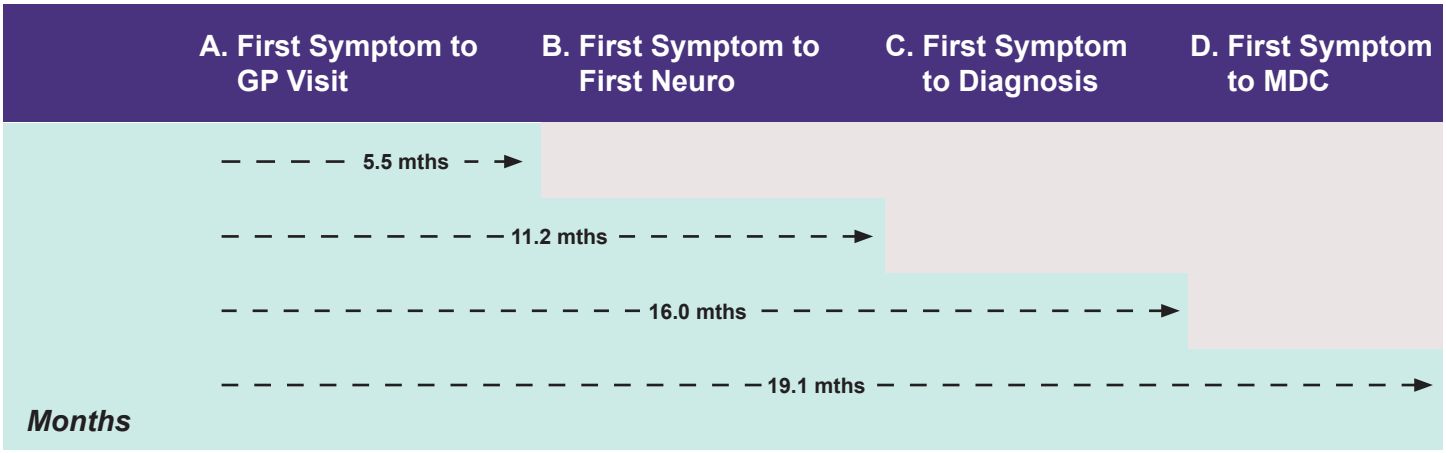

Figure 2: Diagnosis is often delayed by an average of 12 months after symptom onset[5]

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Clinical Considerations and commentary

- Initial treatment plan, involvement of multidisciplinary team[9,10]
- Initial conversation about advance directives[11,12]
- Department of Veterans Affairs has recognized the increased prevalence of ALS in veterans and has classified ALS as a disease presumed to have been caused by military service; veterans who are diagnosed with ALS and served for 90 days or more will qualify for disability services.[13]
- Patients with ALS can apply for Social Security disability benefits, with coverage starting five months after they have become disabled[14,15]
- Clinical progress documentations are essential to obtaining coverage for medications and services to support and justify for insurance authorizations. An example of the required clinical information is shown in Box 1

Required Clinical Information

Medical notes documenting all of the following:

- Drug name and regimen
- Confirmation the drug is being prescribed by or in consultation with a neurologist
- El Escorial/Revised Airlie House diagnostic criteria results that indicate a diagnosis of definite or probable ALS
- ALS Functional Rating Scale-Revised (ALSFRS-R) results prior to treatment
- Forced Vital Capacity (FVC) % at the start of treatment
- Location where the drug will be administered (e.g. infusion center, physician office, self-administered, home health nurse); if the location is in a facility, provide office notes for at least one of the following:
  - Medically unstable based upon submitted clinical history
  - Initial medication infusion of or re-initiation after more than 6 months following discontinuation of therapy
  - Previous experience of a severe adverse event following infusion
  - Continuing experience of adverse events that cannot be mitigated by pre-medications or infusion rate adjustments
  - Physically and/or cognitively impaired and no home caregiver available
  - Difficulty establishing and maintaining patentvascular access
  - Homecare or infusion provider has deemed that the member, home caregiver, or home environment is not suitable for home infusion therapy

Table 1: An example of clinical documentation required for insurance authorization for treatments in ALS

1. Johnson CR. Aquatic Therapy for an ALS Patient. Am J Occup Ther. 1988;42(2):115-120.
2. Desport JC, Preux PM, Truong TC, Vallat JM, Sautereau D, Couratier P. Nutritional status is a prognostic factor for survival in ALS patients. Neurology. 1999;53(5):1059-1063.
3. Wang Z, Wang Z, Fang Q, Li H, Zhang L, Liu X. Effect of Expiratory Muscle Strength Training on Swallowing and Cough Functions in Patients With Neurological Diseases. Am J Phys Med Rehabil. 2019;98(12):1060-1066.
4. Pattee GL, Plowman EK, (Focht) Garand KL, et al. Provisional best practices guidelines for the evaluation of bulbar dysfunction in amyotrophic lateral sclerosis. Muscle and Nerve. 2019;59(5):531-536.
5. Galvin M, Madden C, Maguire S, et al. Patient journey to a specialist amyotrophic lateral sclerosis multidisciplinary clinic: An exploratory study. BMC Health Serv Res. 2015;15(1):1-8.
6. Ferraro PM, Cooke JI, Hennessy L, et al. Multimodal Characterization of Delayed Diagnosis in ALS (S13.009). Neurology. 2019;92(15 Supplement):S13.009.
7. Roggenbuck J, Quick A, Kolb SJ. Genetic testing and genetic counseling for amyotrophic lateral sclerosis: An update for clinicians. Genet Med. 2017;19(3):267-274.
8. Karam C, Berry JD. Heterogeneity, urgency, generalizability, and enrollment: The HUGE balance in ALS trials. Neurology. 2019;92(5):215-216.
9. Hogden A, Foley G, Henderson R, James N, Aoun S. Amyotrophic lateral sclerosis: improving care with a multidisciplinary approach. J Multidiscip Healthc. 2017;Volume 10:205-215.
10. Stephens HE, Young J, Felgoise SH, Simmons Z. A qualitative study of multidisciplinary ALS clinic use in the United States. Amyotroph Lateral Scler Front Degener. 2016;17(1-2):55-61.
11. Kluger BM, Persenaire MJ, Holden SK, et al. Implementation issues relevant to outpatient neurology palliative care. Ann Palliat Med. 2018;7(3):339-348.
12. Levi B, Brothers A, Whitehead M, et al. Advance care planning decision aid improves clinician understanding of wishes of patients with ALS. BMJ Support Palliat Care. 2012;2(2):175.1-175.
13. Compensation and Pension Service. Disability compensation: "presumptive" disability benefits. US Department of Veterans Affairs website. benefits.va.gov/BENEFITS/factsheets/serviceconnected/presumption.pdf. Published December 2017. Accessed January 9, 2020.
14. Social Security Administration Program Operations Manual System (POMS). DI 23022.100 amyotrophic lateral sclerosis (ALS): compassionate allowance information. Social Security Administration website. policy.ssa.gov/poms.nsf/lnx/0423022100. Reviewed October 24, 2008. Accessed on January 9, 2020.
15. What you need to know when you get Social Security disability benefits: publication no. 05-10153. Social Security Administration website. ssa.gov/pubs/EN-05-10153.pdf. Published January 2017. Accessed January 9, 2020.

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