

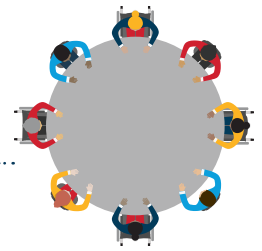


ALS ASSOCIATION NOVEMBER 2020 ROUNDTABLE MEETING ON REDUCING ALS COMPLICATIONS

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November 18-19, 2020

OVERVIEW



ALS ASSOCIATION ROUNDTABLE PROGRAM OVERVIEW

On November 18 and 19, 2020, The ALS Association (the Association) convened our fourth multi-stakeholder Roundtable. This meeting focused reducing medical complications associated with ALS, such as pneumonia, bedsores, pain, or discomfort. The roundtable met virtually over two consecutive half-days, with more than 75 people participating in two plenary sessions and a total of three small-group break-out sessions. In addition to detailed presentations and discussions on complications, participants also received updates on:

- Reducing Time to Diagnosis (first discussed at the spring 2020 Roundtable meeting)
- Access to Future Therapies (first discussed at the fall 2019 Roundtable meeting)
- The Association’s ongoing strategic planning process

The Roundtable discussions were enlightening and intense. Our conversations were among the first to focus on these issues in a multi stakeholder format. Based on these conversations, we believe it is possible to reduce the frequency of these complications and improve experience of people living with ALS, and their families.

Since launching in 2019, the Association’s Roundtable Program has regularly assembled members of the ALS community for candid, facilitated discussions about priorities for people with ALS, helping shape the Association’s strategic planning efforts, and identifying action steps to improve ALS care, advocacy, and research. Roundtable meetings convene a diverse group of individuals reflecting a range of ALS expertise and experiences, including people living with ALS, caregivers, clinicians, and industry partners.

While the Roundtable Program launched with in-person gatherings, the COVID-19 pandemic prompted the 2020 Roundtable sessions to “go virtual.” Though the format has changed, there continues to be a high level of engagement, participation, and productivity among participants.

2020 Roundtable Program sponsors include: Alexion, Amylyx Pharmaceuticals, Apellis, Biogen, Biohaven Pharmaceuticals, Cytokinetics, Helixmith, Ionis Pharmaceuticals, Novartis Gene Therapies, Orphazyme, and Mitsubishi Tanabe Pharma America. The meeting was facilitated by Ilisa Halpern Paul, president of the District Policy Group at Faegre Drinker Biddle. Our discussion was also shaped by contributions from several people with ALS and their caregivers, and leading scientists and ALS clinicians.

SPOTLIGHT ON ALS COMPLICATIONS: BACKGROUND & CONTEXT

People with ALS face a multitude of physical, psychological, emotional, and financial challenges as they navigate their disease journey. This journey is often complicated by

other conditions associated with ALS. Such complications (also referred to as symptoms that result from disease progression) often lead to emergency department visits, inpatient hospitalizations, and even death. They cause pain, distress, discomfort, fatigue, reduction in quality of life, and negative financial impact for people with ALS and their families.

The Association has identified reducing and preventing complications from ALS, and thereby improving the lives of people with ALS and their caregivers, as a top near-term priority.

We spent the first day of the roundtable on enumerating the top complications and symptoms associated with ALS and setting the stage for smaller breakout discussions on the second day. Presenters and panelists represented the key stakeholder communities, people with ALS, caregivers, clinicians, researchers, and industry partners.

We considered the perspectives of clinicians, people with ALS, caregivers, and trial sponsors as we focused on three questions in their discussions throughout the meeting:

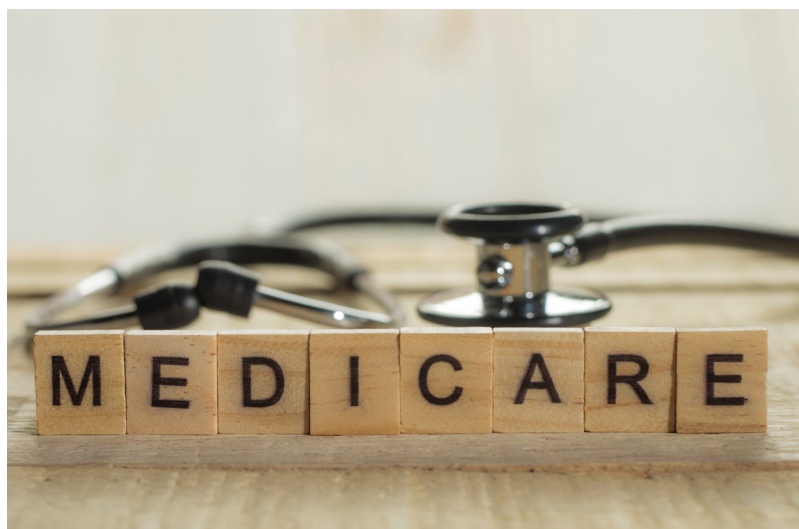
1. What are the complications that should be the focus of our efforts in this arena?
2. What interventions can ameliorate those conditions? How can we measure if those interventions are working?
3. What else needs to be done/should we do with respect to complications of ALS?

The next sections of this report summarize the plenary and panel discussions held on the first day of the roundtable and the recommendations, themes, and issues identified in the three breakout discussions on the second day.

REDUCING COMPLICATIONS KEY TAKEAWAYS:

MEDICARE CLAIMS DATA ON COMPLICATIONS

- We conducted a review of a nationally representative sample from Medicare Fee-For-Service (FFS) claims data from 2016 to 2019 indicates that most of the people with ALS in the U.S. are covered by Medicare (either as their primary coverage, in conjunction with Medicaid (people who are dually eligible), or as secondary coverage to private insurance).
- People with ALS who are under 65 years of age can qualify for Medicare through Social Security Disability Insurance (SSDI). There are approximately 16,000 people with ALS in Medicare FFS each year.
- Medicare FFS beneficiaries with an ALS diagnosis go to emergency departments (ED) at higher rates than Medicare FFS beneficiaries without an ALS diagnosis.



OVERVIEW



- Those Medicare FFS beneficiaries with an ALS diagnosis who do present to ED are admitted to the hospital for an inpatient stay more frequently than their Medicare FFS beneficiary counterparts without an ALS diagnosis.
- By comparing the top reasons for ED visits and inpatient hospital stays among people with ALS in Medicare to the general Medicare FFS population, we were able to isolate the most frequent serious complications for people with ALS. These include sepsis, pneumonitis due to inhalation of food and vomit, acute and chronic respiratory failure, urinary tract infection, and gastrostomy malfunction. Of note, beyond the top five complications: falls also factor into more ED visits for the ALS Medicare FFS population in comparison to the general FFS Medicare population.

CLINICIAN/CARE TEAM PERSPECTIVE ON COMPLICATIONS

- Several leading ALS clinician scientists presented their perspective on top complications of ALS, which they categorized as: infections, respiratory muscle movement, pulmonary embolism, gastrointestinal, difficulty swallowing, weakness/immobility/spasticity, and cognitive issues (Figure 1).
- Within these categories there is often a cascading effect without proactive intervention, where one problem can lead to multiple compounded problems with increasing severity. With the right interventions, it is possible to reduce the frequency of these complications.

Figure 1: List of ALS Complications identified.

ALS COMPLICATIONS

- Infections
 - Urinary Tract Infections
 - Pneumonias
 - Line Infections
 - Stomal Infections (trach or feeding tube)
 - Decubit (bed sores)
 - Sepsis
 - Infection and inflammatory reaction to cystostomy catheter
- Respiratory Muscle Involvement
 - Dyspnea
 - Acute and chronic respiratory failure
- Pulmonary embolism
- Gastrointestinal
 - Constipation
 - Gastronomy malfunction
 - Malnutrition
- Dysphagia (difficulty swallowing)
- Weakness, Immobility and Spasticity
 - Falls
 - Contractures
 - Stiff Shoulder
 - Cramps
 - Cognitive-FTD

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