LIVING WITH ALS

RESOURCE GUIDE 1

# What is ALS?

## An Introductory Resource Guide for Living with ALS

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ALS ASSOCIATION

# WHAT IS ALS?
# AN INTRODUCTORY RESOURCE GUIDE FOR LIVING WITH ALS

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ALS ASSOCIATION

***A note to the reader:*** The ALS Association has developed the Living with ALS resource guides for informational and educational purposes only. The information contained in these guides is not intended to replace personalized medical assessment and management of ALS. Your doctor and other qualified health care providers must be consulted before beginning any treatment.

**Living with ALS**
**What is ALS? An Introductory Guide for Living with ALS**

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## INTRODUCTION

Learning that you or a loved one has Amyotrophic Lateral Sclerosis (ALS) can be overwhelming. ALS is a complex disease and there is much to learn. Most likely, after processing this diagnosis in your own mind, you will decide how much information about ALS you want to know and when you want to know it. Some people want to know as much as possible from the time of diagnosis, while others prefer to learn as changes take place. Both ways are appropriate and will allow you to implement solutions and options for maximizing functional independence. This introductory resource guide is one of several published by The ALS Association. It includes the basics, beginning with how ALS is diagnosed, how the disease affects a person over time, and how it can be managed as it progresses.

What we will cover in this resource guide:

- What the three letters of "ALS" mean
- The role motor neurons play in how our bodies move
- Facts about ALS
- How ALS is diagnosed
- How ALS progresses
- Treatment and symptom management for ALS
- How an ALS care team can help
- ALS research and clinical trials

When you are ready to learn more, you will find additional detailed information on specific topics associated with living with ALS in the other ALS Association resource guides available. These guides can be ordered in print from The ALS Association or can be downloaded online at www.alsa.org.

## WHAT DOES AMYOTROPHIC LATERAL SCLEROSIS (ALS) MEAN?

ALS is a type of **Motor Neuron Disease (MND)**. MNDs are diseases that damage the motor nerve cells, which are cells required for movement. **Amyotrophic** is a medical term meaning loss of nutrition to the muscle. Muscles lose their bulk and get smaller. The term **sclerosis** means scarring or hardening. In ALS, this scarring is due to the damage and loss of nerve cells. **Lateral** means the side and refers to the area of the spinal cord that houses the fibers of the nerve cells that die off in ALS.

ALS causes weakness and wasting of all **voluntary muscles**. This means that the muscles we use to move, swallow, and even breathe, become affected by ALS. The disease can start in different places in the body. However, as time progresses, the weakness worsens in the muscles that were affected first and then spreads to other parts of the body.

**Unfortunately, there is no cure for ALS.** There is no known way to stop or reverse this disease. **There are, however, treatments** that ALS specialists recommend to help people manage their symptoms. The ALS Association's resource guides

are meant to help you and your loved ones understand this disease and live fully with the diagnosis. Please know that your ALS healthcare providers are also available to help you navigate the challenges that this disease brings.

### ALS and "Lou Gehrig's Disease"

In North America, ALS is sometimes referred to as Lou Gehrig's disease. As baseball fans know, Lou Gehrig, known as "The Iron Horse," was a powerful baseball player for the New York Yankees in the 1920's and 1930's. While playing baseball, he noticed that he was becoming weak with no clear reason. He was evaluated at the Mayo Clinic and diagnosed with ALS. Lou gave his famous farewell speech on July 4th, 1939. Today, ALS is still commonly referred to as Lou Gehrig's disease. People with ALS and healthcare professionals recognize that even strong, healthy people can be affected by this illness.

## THE ROLE MOTOR NEURONS PLAY IN HOW OUR BODIES MOVE

All body movements require nerve cells in the brain and in the spinal cord. These nerve cells are called **motor neurons** and they control our muscles. The brain sends signals to the appropriate nerve cells in the spinal cord that control the muscles in the arms, legs, and feet, for example, to perform actions such as picking up a glass or moving a foot. These cells send messages to the muscles via a long nerve fiber called an **axon** (**Figure 1**).

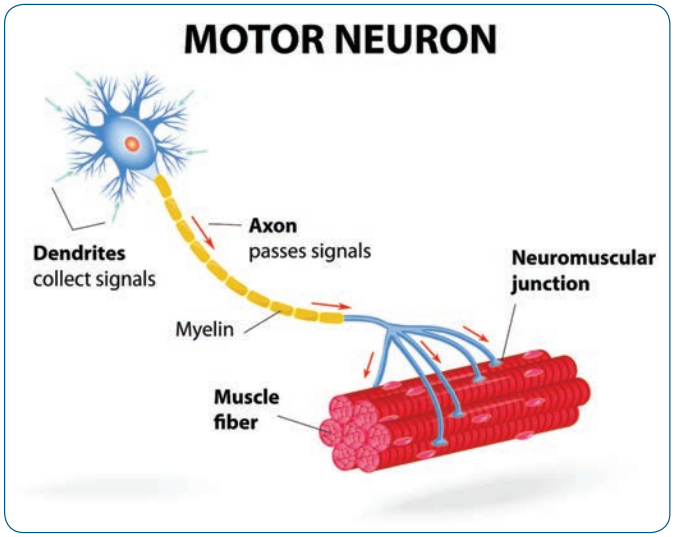


**Figure 1:** A motor neuron and its axon.

Without this signal, there is no way for a muscle to know what to do. It is similar to computer keyboards: if they are not plugged in, there is no connection and the computer screen remains blank. The muscle also needs this nerve connection to survive.

There is a **symbiotic relationship** (that means they need each other) between every muscle in our body and a specific nerve axon. If the connection is severed,

not only will the muscle not get the signal of what to do, it also will shrink (medical term: **atrophy**) without an axon (**Figure 2**). This is why, in ALS or other MNDs, we cannot stimulate the nerve artificially to reverse the effects of the disease: **the axon must be intact for the muscle to remain healthy.**

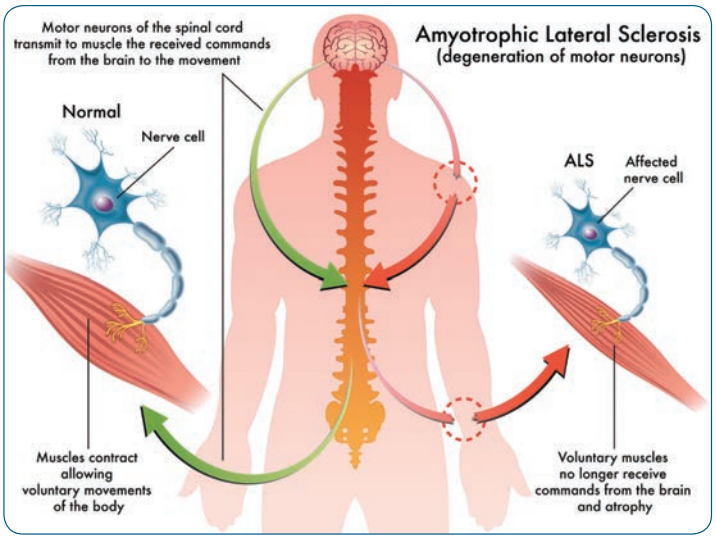


**Figure 2:** Muscle wasting as a result of motor neuron disease.

## Voluntary Movement (What's Involved and What's Not)

In our bodies, we have three types of muscles: striated, smooth, and cardiac. In general, all striated muscles are **"voluntary,"** meaning we directly control their movement with our thoughts. In ALS, the striated muscles that move the arms and legs, as well as the muscles of the back and neck, can become affected. The muscles we use to speak are also striated and voluntarily controlled and, therefore, can become affected.

But there are some subtle exceptions. The respiratory center in the brainstem is responsible for controlling a person's breathing rate. It automatically sends a message to the respiratory muscles telling them when to breathe. The main breathing muscle is the diaphragm, which is a striated muscle. This control is involuntary and continuous, meaning you do not have to consciously think about it. **Breathing, however, is most unique compared to other involuntary functions (**medical term: **autonomic or visceral functions like digestion) in that it can also be regulated voluntarily.** Every person with ALS has a different pattern to start, but eventually, all these muscles can be affected.

The muscles that control the eye movements (medical term: **extraocular muscles**) and the muscles that guard and control the opening of the bowel and bladder (medical term: **voluntary sphincters**) are striated muscles, but are affected very late or sometimes not at all in the disease. It is not known why these eye muscles and voluntary sphincters are less affected.

The heart muscle (medical term: **cardiac muscle**) is also not affected. Smooth muscle, such as the food pipe (medical term: **esophagus**) or bladder are generally

not affected, but weakness and spasticity can make toileting difficult and cause leakage of urine (medical term: **urinary incontinence**).

# FACTS ABOUT ALS

## What Are ALS and MND?

The term amyotrophic lateral sclerosis is not a very good description of the disease, because we know the problem is **not** nutrition to the muscle, but rather death of the nerve that connects to the muscle. However, clinicians and people in North America still use the term ALS because it is so well known and more specific than MND.

As mentioned, another term for ALS is "motor neuron disease," or MND. This is a better description of what happens in the body: the nerve cells that communicate (innervate) with muscles are diseased and die. However, this term, MND, is used less often because there are other causes of MND besides ALS (i.e., MND does not refer only to people with ALS). ALS is a type of MND. Other types of MND include progressive bulbar palsy, primary lateral sclerosis, and progressive muscular atrophy.

## When and How Likely Is It for ALS to Occur?

ALS most commonly occurs between the ages of 40-70, but it can occur at much younger and older ages as well. Men are slightly more affected than women. ALS is a rare disease that, on average, affects about 6 in 100,000 people at any given time (**prevalence rate**). The number of newly diagnosed cases per year is approximately 2 per 100,000 people (**incidence rate**). The research and data collected annually through the **National ALS Registry** provides more current information on the actual number of people who have ALS in the United States.

## What Causes ALS?

ALS most often occurs **sporadically**, meaning without a known cause or warning. There is no known direct cause. Current research shows no correlation to diet, exercise, activities, or jobs. There is a relationship between military service and smoking leading to a higher likelihood of getting ALS, but it is unclear if either military service or smoking actually causes ALS. Research is being done to figure out what factors in the environment may influence the likelihood of developing ALS, but there have not been any valid findings to date. Occasionally, there are rumors of 'ALS triggers' but none have yet been proven.

## Forms of ALS (Sporadic ALS, Familial ALS)

**Sporadic ALS** is responsible for approximately 90% of all cases of ALS diagnosed. Sporadic means the disease is not passed down in families. Researchers are looking for genes that may make one person more likely than another to develop ALS.

There is a small group of people with a genetic form of ALS referred to as **familial ALS** or **fALS**. This makes up approximately 10% of people with ALS and it has a high presence in the family (medical term: **penetrance**). This means that many family members are affected by it (e.g., parents, siblings, grandparents; not typically a second or third cousin or remote family member). However, if you have concerns about a family connection, you should speak to your physician or healthcare provider to discuss the potential of developing fALS and any recommended genetic testing. (See the genetics fact sheet published by The ALS Association for more information.)

### Genetics and ALS and FTD

Even though we have just identified ALS as "sporadic" in the majority of cases, researchers and clinicians are beginning to see a pattern with other diseases and genes that are associated with ALS. One of these conditions is **Frontotemporal Dementia**, or **FTD**.

FTD is a rare form of dementia that causes trouble with decision-making and sometimes changes in behavior (e.g., saying inappropriate comments or not responding to emotions). There may be a common link to both ALS and FTD. It is believed that up to 50% of people with ALS may have some mild changes in decision-making and behavior (medical term: **frontotemporal dysfunction**), but not outright FTD. You and your caregivers may want to discuss this with your physician or healthcare provider if you notice any of these symptoms. For more information, see the resource guide, *Changes in Thinking and Behavior in ALS.*

## DIAGNOSING ALS

ALS is difficult to diagnose because there are no clear triggers and in most cases, there are no specific genes. It is diagnosed by asking disease-related questions (medical term: **history taking**) and examining the person (medical term: **physical examination**). Thus, being evaluated by a neurologist with experience in ALS is very important. There are certain symptoms and examination cues that need to be seen together to confirm the diagnosis of ALS and, just as important, rule out other possible conditions. **There is no specific test for ALS.** ALS is a diagnosis of exclusion. This means that physicians order various tests to look for another explanation (rule out other potential diagnoses). Some tests that may be ordered include blood work, an Electromyogram/nerve conduction study (also called **EMG**), and Magnetic Resonance Imaging (**MRI**). None of these, however, is required to make the diagnosis of ALS. It is not unusual to think of the first weakness associated with ALS as a symptom of something different. For example, many people are diagnosed with pinched nerves before ALS is considered. It often takes up to a year from the first symptom before the diagnosis is confirmed.

ALS is diagnosed when physical and laboratory examinations indicate that a person has weakness caused by the loss of motor neurons, which worsens over time (medical term: **progression**) and spreads to different parts of the body (medical term: **multiple regions**). First, the neurologist looks for signs that there is

motor neuron damage. There are two different types of motor neurons that cause very characteristic findings when they are damaged: upper motor neurons and lower motor neurons.

**Upper Motor Neurons** (UMN) are nerve cells that travel from the brain to the spinal cord to control movement. When they are lost, you have stiffness of the limb (medical term: **spasticity**), brisk reflexes (medical term: **hyperreflexia**), and weakness.

In addition, as we mentioned earlier, you will also have loss of motor neurons in the spinal cord (the **lower motor neurons**). These are the nerves that run from the spinal cord to the muscle. Loss of the Lower Motor Neuron (LMN) causes weakness, muscle wasting (medical term: **atrophy**), and muscle twitching (**fasciculations**).

**Fasciculations** are spontaneous twitches or movements of parts of a muscle, not the entire muscle. It is not a jerk, but a painless fluttering within the muscle. There are many causes for fasciculations (fatigue, exercise) and not all fasciculations are due to ALS. They are, however, symptoms associated with this disease. Fasciculations are not harmful; they are simply signs of irritation of the lower motor neuron.

Neurologists who diagnose ALS divide the body into four regions based on the way the nerves that attach to muscles are wired: 1) the face and tongue (**brainstem**); 2) the arms (**cervical**); 3) the trunk (**thoracic**); and 4) the legs (**lumbar**). The neurologist looks for signs of upper motor loss (spasticity and hyperreflexia) and lower motor neuron loss (atrophy and fasciculations) in at least three of these four regions of the body (**Figure 3**) to determine if a person has ALS.

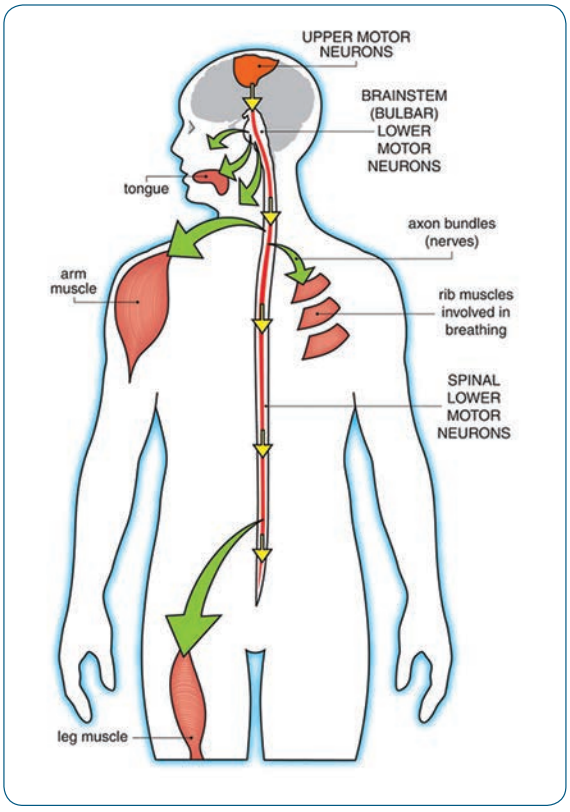


**Figure 3:** Upper and lower motor neuron connections in the brainstem (face and tongue), cervical (arms), thoracic (trunk), and lumbar (leg) regions (Source: cpchildrentreatment.com)

**It is not unusual to seek a second opinion after you have been given an ALS diagnosis.** Many doctors will refer you to a specialized clinical center to make sure nothing was missed. It can take up to three or four visits to come to terms with the diagnosis. It is important that you find a provider you can trust, and feel comfortable with the care you receive.

*My diagnosis hit me like a brick. For the next 3 months, I couldn't talk about it with anybody but my wife. After getting my third opinion with the same diagnosis, I knew I had to come to grips with the situation and take advantage of the life I had left. We joined a support group to share feelings with people just like me, traveled and just looked for the good in every day.*

Jay DePaola (Contributed by The ALS Association Greater New York Chapter)

## Common Reactions to the Diagnosis

It is normal to experience shock and disbelief when you are diagnosed with ALS. This is normal … no one expects to ever have a disease like this. It is important to pay attention to your emotions and talk about your feelings and fears. Our emotional health can affect our physical wellbeing.

It is also important to remember that you are not in this alone. Your physician, healthcare providers, and representatives from your local chapter of The ALS Association can help you sort through your feelings and process the changes in your life. Some individuals with ALS find comfort in a support group setting while others turn to online forums. It can also be helpful to meet other people dealing with the same illness.

*I was partly relieved to finally have an explanation for all of the strange symptoms I'd been experiencing, but also deeply saddened when I thought of the burden I would be placing on my spouse and my children.*

Daniel (Contributed by The ALS Association Golden West Chapter)

*After being diagnosed, changes to my body became more noticeable and seemed to come faster. The first noticeable change was my balance. I would lose my balance much more easily and fall. This made taking care of two children under the age of two extremely difficult and frustrating. Within a year of being diagnosed, I was using the wheelchair you see me in now. I could still walk with a walker, but the chair was much faster. Not only was I changing physically, but also emotionally and mentally. I was angry, frustrated, and so much more. I unwittingly pushed people away. Even the ones I loved most. I was angry because I was losing the use of my body. I was an athlete, a soldier, a husband, and a father. All of that was changing forever.*

Excerpt from Troy Musser Rockwell Speech
(Contributed by The ALS Association Iowa Chapter)

*It's funny, but when I was given my ALS diagnosis, I did not fall to pieces or want to give up. In my thinking, I see it as a journey to challenge me. It may be hard, but there are blessings along the way. After all, everyone's time on earth is limited and we must all make the most of what we have. This experience has drawn my husband and me closer. I appreciate all the help he gives me and I try to remember to always thank him. I see more of my children and grandchildren (a big plus for me), and another thing: I am amazed at the thoughtfulness and kindness of people when I go out to shop or eat. Most times when I am approaching a door, people will smile, speak, and hold the door. They will help me be seated and even move my walker or rider to a vacant corner. It's amazing, especially when the person is sitting at a table and just about to take a bite of food. They will put down the fork and spring for the door. It's kind of funny, but such a huge blessing for me.*

Donnie Stell (Contributed by The ALS Association North Carolina Chapter)

## HOW DOES ALS PROGRESS?

Many people want to know how quickly ALS progresses. This is another difficult aspect of this disease as **no two individuals present exactly the same**. Some people have severe involvement in one area before it spreads, while others experience rapid progression throughout their body. In some people, the disease progresses very slowly.

In general, people with ALS live about three to five years after they experience the first sign of weakness. This is a generalization, which is based on averages. People with ALS can live anywhere from a few months to decades depending upon disease changes and the types of medical care and assistive devices they choose. **ALS is different in each person and will run an individual course.**

### Symptoms

A person with ALS may develop severe **weakness** (medical term: **paralysis**) of all muscles in the arms and legs and the muscles of breathing, swallowing, and speaking. However, every person is different. Some people have severe weakness of one area, but little in others (e.g., unable to swallow but still able to walk and drive), but other individuals may demonstrate a similar severity of involvement of different areas. Some patients have the disease that progresses very slowly, while others have changes that happen more quickly. **Clinicians have difficulty predicting how a person's ALS will progress at the time the diagnosis is made; therefore, they continually see the individual and assess symptoms with each visit.**

**Your ALS physician cannot fully predict the time course of the disease and cannot entirely predict in what order the body will be affected.** Although no ALS healthcare provider can know for sure, it is generally true that individuals who experience a more rapid onset will have a more rapid course of the disease, while individuals with a slow onset will likely experience slow progression. In general, people with face and tongue (medical term: **bulbar**) involvement have a shorter life span due to the problems with loss of function in this area (breathing,

swallowing). It is also generally true that the spread occurs from one body part to the next. So for instance, if the disease starts in the legs it would be expected that the arms would be affected next.

**Symptoms do not begin all at once or suddenly**. Many people fear that they will wake up paralyzed, but symptoms do not change overnight. Some family members may notice an abrupt change, but this is more often due to the person attempting to compensate (counteract) the gradually worsening weakness until the muscle can no longer support an important function in daily life (for instance climbing stairs or getting up from a chair). It is important to take notice of any changes. Discuss them early with your ALS specialists because they may be able to help treat the symptoms and prepare you for upcoming changes.

Eventually, you will likely need help with most movements, including getting out of bed, moving onto a chair, getting dressed, showering, eating, and toileting. This is due to the loss of the motor neurons causing paralysis.

People **lose muscle mass** and as a result, **lose body weight**. Weight loss is also due in part to increased need for calories, decreased ability to eat adequately because of swallowing difficulties, as well as arm and hand weakness, which impacts the ability to feed oneself.

Some of the symptoms you may experience include difficulty with:

- Going up and down stairs
- Getting up from a chair
- Playing sports
- Moving onto and off the commode (toilet)
- Walking (especially on uneven surfaces) and occasional falls
- Lifting items or holding a pen
- Holding a glass
- Typing on a keyboard or using eating utensils
- Swallowing liquids or foods
- Sleep (poor sleep can result from stress, inability to get into a comfortable position, or from difficulty breathing)
- Breathing (shortness of breath) with activity or when lying down
- Speech (being poorly understood because of slurring words or speaking slowly)
- Decision-making or planning
- Crying or laughing too much (medical term: **pseudobulbar affect**)

The symptoms listed above are discussed in more detail in the other resource guides published by The ALS Association. The following suggestions may provide guidance or an important way of approaching ALS after diagnosis:

- Do not hesitate to make notes about any problem you notice and discuss them with your ALS care team specialists. No problem is too small or unusual to bring up.

- ALS symptoms do not change suddenly, but progress with time. Acknowledging your symptoms when first noticed and addressing them right away will alleviate stress.

## TREATMENT AND CARE FOR ALS

### Rilutek®

As we discussed, ALS is a very complex disease with no cure at this time. There are many researchers investigating this disease but, as of now, there is no way to stop or reverse the progression.

**There is one approved medicine available for people with ALS, called riluzole** (trade name: Rilutek®). It is a 50 mg pill, taken twice a day. It can be taken at any point in the disease and it **has been shown to slow disease progression and prolong life by three months, on average.**

**What does this mean?** This means, in large, randomized clinical trials, people who took the medicine lived an average of three months longer than those who did not take it. It does not mean any one person will "buy" three months when they take it or are guaranteed to live three months longer. It means **the drug appears to moderately slow the progression of the disease and increase the likelihood to survive a year longer by 10%.** It appears to work better in people who are older or who have the disease start in the face and tongue muscles (bulbar ALS).

**Some people with ALS may say, "Why take a medicine with so little effect?"** Most people tolerate the medicine well and want to feel as though they are doing something to fight the disease. The medicine has very few side effects but people still need to have blood work done every three months to monitor the effects of the medicine. Choosing to take riluzole is a personal decision to be discussed with your physician.

Ongoing research with the participation of people living with ALS is looking at ways to improve ALS treatments (medical term: **clinical trials**). Not all trials are available to every person and not all trials are available at every medical center. If you are interested in participating in clinical trials, you may wish to discuss this with your ALS physician during an appointment. Using the drug riluzole to slow down the progression of ALS will not keep you from participating in clinical trials.

**As of this printing, there are no other treatments that have been proven to cure or reverse ALS.** Well-meaning friends and family may hear of someone who was "cured" by a special treatment that was found on the internet or that required traveling abroad. These are, unfortunately, unproven claims and just rumors. A good website to check the validity of these claims is **ALS Untangled** at http://www.alsuntangled.com. **To learn more about clinical trials in ALS, please see the section at the end of this resource guide.**


## The Care Team

It takes a team to care for you and to help you manage ALS. Accepting help and care can be difficult at first. Worrying about being dependent or being "a burden" to others are very common thoughts. Remember that your family and friends want to help you, and you accepting help makes a difference in managing ALS. A care team that works together and supports each other makes the biggest impact every step of the way.

**The most important member of the care team is you, the person with ALS. It is you who sets the priorities and makes the decisions.** Your team also includes caregivers such as spouses, parents, or children. It may include other relatives, friends, neighbors, and other members of the community. There is usually one person who is considered the main caregiver due to being more intimately involved and they are often in charge of the day-to-day activities and planning.

As mentioned above, in some people, ALS can affect the ability to make decisions. Your healthcare team will assess you for this possibility since it may impact the way you navigate the disease. If there is concern about your decision-making, it becomes very important to identify a trusted person who can act on your behalf.

You can read more about how ALS may affect thinking and decision-making and about how you can plan your care in the dedicated chapters of this resource guide.

The medical care team includes many specialists. **Ideally, the person with ALS has access to a Certified Treatment Center of Excellence, a Recognized Treatment Center or clinic with expertise in ALS, which includes an interdisciplinary care team.** The benefit of having a professional interdisciplinary team comes from getting coordinated care from multiple healthcare disciplines. The members of the team collaborate with each other to ensure that every person has the same information and works toward common goals and priorities that the person with ALS has set. This approach ensures that care is delivered in a way that puts the patient and family at the center.

*Coming to an ALS Association Certified Treatment Center of Excellence every three months allows us the opportunity of learning about the progression of our disease, whether the progression has not occurred or whether it is getting worse. We would not know this if we didn't go to a Certified Treatment Center of Excellence. At Certified Treatment Centers of Excellence, we have the opportunity of seeing a multidisciplinary team of experts who let us know what to do to keep our physical and emotional condition at its very best. Without their expertise, we would not fully understand where we stand in our disease progression. It provides us with a sense of reassurance.*

Cary Marsh (Contributed by The ALS Association Greater New York Chapter)

## Certified Treatment Center of Excellence Program

The ALS Association has a **Certified Treatment Center of Excellence Program** that helps provide care for people with ALS and their families. A certified center has all the required medical professionals available to provide the best care possible, including:

- **Neurologist** (medical doctor who specializes in diseases of the brain, nerves, and muscles and is experienced in treating ALS)
- **Nurse or Nurse Practitioner (RN or NP)**
- **Respiratory Therapist (RT)**
- **Physical Therapist (PT)**
- **Occupational Therapist (OT)**
- **Speech-Language Pathologist (SLP)**
- **Dietitian (RD)**
- **Social Worker (SW)**
- **Mental health professional**
- **ALS Association chapter liaison**

*My husband and I began making quarterly visits to an ALS clinic shortly after he was diagnosed with ALS. We could not have gotten through this traumatic time in our lives without the encouraging support from the dedicated team there. Their tests, evaluations, and support have been invaluable in guiding us through this devastating illness.*

Valerie Torretti, wife of Gary Torretti, who is living with ALS
(Contributed by The ALS Association Greater Philadelphia Chapter)

Many centers offer the services of a **pulmonologist** (doctor specializing in the respiratory system), **gastroenterologist** (specializing in the digestive system), **palliative care physician** (specializing in supportive care and pain management), **physiatrist** (specializing in physical and rehabilitative medicine), **psychologist and other mental health professionals** such as **psychiatrists** (specializing in assessment of thinking and mental health).

*We learned a lot of various aspects of dealing with ALS when we attended the Treatment Center of Excellence, ranging from nutrition to speech therapy. Every time we go back, armed with questions as our situation evolves, we learn more helpful approaches to dealing with the disease.*

Judith Massey (Contributed by The ALS Association Northern Ohio Chapter)

With a certified treatment center, instead of making separate appointments in different places with all the professionals and services needed, there is a single clinic visit for all the different specialists you need to see. The clinic visit may be long and somewhat tiring, but usually, people feel it is worthwhile and productive. Many people will bring family members and friends to these visits, so everybody can ask questions and learn from all the care providers.

*I have received excellent care from the VA ALS Clinic. The clinic combines all disciplines all at once to see us in the same day, rather than multiple appointments on multiple days. Some of those disciplines we see are respiratory, physical therapy, occupational therapy, and several others. It makes for a long day, but only one day.*

Excerpt from Troy Musser Rockwell Speech
(Contributed by The ALS Association Iowa Chapter)

Research studies suggest that getting **care in an interdisciplinary ALS specialty setting allows for better quality of life and may extend life.**

For some people with ALS, attending an ALS Certified Center of Excellence Program may not be possible due to distance, physical difficulties in travel, or poor health. Please discuss any challenges and your specific needs with your ALS care team or your local ALS Association chapter. These specialists may be able to offer telehealth services to your home (using video/telecommunication or other technology to communicate with your healthcare team) or provide appropriate local referrals, support, or suggestions to help you maintain your plan of care

based on your goals and priorities. Your local primary care physician may also be able to connect with a specialized ALS team in order to receive guidance and access resources or may choose to enlist the help of a local palliative care or hospice care team.

## Clinical Trials

Clinical trials are research studies with volunteer participants. **The goal of clinical trials is to answer specific scientific questions to find better ways to prevent, detect, or treat ALS, or to improve care for people with ALS.** Clinical trials geared to finding better treatments usually occur after research is done with animals or other testing methods in a laboratory. **Table 1** shows the goals of different types of clinical trials.

Once a treatment is to be tested in people, the testing is usually done in several stages:

1. The first step is called a **Phase 1** study and involves only a small number of participants. The purpose of Phase 1 is **to see how a drug may affect the human body (e.g., side effects) and what range of dose may be used.**
2. The next step is a **Phase 2** study. The focus of Phase 2 is **to see further if the drug is safe and tolerated** and to get a better sense of dosing and possible effects, in a larger group.
3. The last step is a **Phase 3** study, which usually involves a fairly large group of participants. The purpose of Phase 3 is **to determine if the new treatment is working and if it is better than any of the known and available treatments.**

**Table 1:** Types of Clinical Trials

| |
|---|
| Treatment (slowing down, halting or reversing the progression of disease) |
| Prevention (avoiding the start of the disease) |
| Screening and early detection (finding out who will get the disease) |
| Diagnostic (confirming that somebody has the disease) |
| Genetics (investigating how the disease may be inherited or caused) |
| Quality-of-life / supportive care (finding best treatments for symptoms) |

Clinical trials follow a recipe or a blueprint called a **study protocol**. It outlines the purpose of the study, how many people will participate, who is able to participate, what will be done, and what information will be gathered about participants.

One of the most important parts of the study protocol is **eligibility**. Eligibility criteria outline the attributes a participant must have to be part of the study. They are worded as attributes that one must have (criteria for inclusion), as well as attributes that need to be avoided (criteria for exclusion). Usually, there has to be a reasonable suspicion that a person indeed has ALS. The person also has to be able to understand and agree to be part of research. **Having advanced stages of ALS, using a ventilator, or living far away from the research center often make participation in a clinical trial difficult and may result in exclusion from participation.** The purpose for inclusion and exclusion criteria is to ensure that people who participate in the study are sufficiently similar in respect to their disease. The reason why this is important is that it helps determine if what is assessed in the study (i.e., treatment method) is truly associated with a change in the condition or outcome (i.e., living longer) without being influenced by differences between participants.

The same clinical trial is usually carried out by teams of researchers at different places throughout the country and the world. The main researcher who designed the study and oversees the research is referred to as the **principle investigator**.

**Most clinical trials are conducted in what is called a double-blind placebo-controlled method.** Placebo-controlled refers to the fact that participants are divided randomly into two groups. One group will receive the actual intervention (like a new drug or medical device). The other group will be given something that tastes, looks, or feels similar, but does not have any effect. Double-blind refers to the fact that neither the researchers nor the participants know to which group the participant has been assigned until the study is concluded and the results are analyzed. This arrangement helps avoid measuring an improvement that is only seen because a person has the expectation or belief that a treatment is helping (**placebo effect**).

**It is important that everybody who is affected by ALS considers helping with research efforts.** This can be as easy as registering through the National ALS Registry and completing questionnaires or as complicated as getting a medical device implanted. There are also opportunities for research that include caregivers and other members of the care team.

**The best place to look for up-to-date information on ALS clinical trials is** www.clinicaltrials.gov. You can select ALS as the target disease and then narrow the search by geography by entering a zip code or by type of trial and recruitment status. You will find a detailed description of the trial and contact information. You may also visit the website of The ALS Association to look for listings of ongoing trials or research results. The Northeast ALS Consortium (NEALS) is another source of good information (www.alsconsortium.org).

## SUMMARY STATEMENT

ALS is very difficult to understand because it is filled with uncertainty and change. Knowledge is power. By learning about ALS, you can be more prepared when it's time for clinic appointments and you can stay on top of each issue as you tackle it. In this introductory resource guide, we covered the basic facts about what ALS is, its diagnosis, progression, care, and treatment, and the role you can play in research. You can choose what, how, where, and when to learn about all the aspects of this disease that now is a part of your life. You can choose your pace. For more detailed information about living with ALS, refer to the other topic-specific resource guides and additional information available through your local ALS Association chapter or the national office and your ALS clinical team of experts. Use these supports—they are here to help you and your family to make plans and decisions regarding your specific goals and priorities throughout your disease course. The goal is to maximize your quality of life, prevent crises, and remain in control of your healthcare decisions.

The following is a list of topics covered in the *Living with ALS* resource guides:

### *Resource Guide 1*

**What is ALS? An Introductory Guide for Living with ALS**

This resource guide provides an overview of ALS, what it is, and how it affects your body. It provides information on what kind of resources are available to help you deal with ALS more effectively.

### *Resource Guide 2*

**After the ALS Diagnosis: Coping with the "New Normal"**

This resource guide addresses the psychological, emotional, and social issues that you must face when your life is affected by ALS. It provides information on how to cope with the many lifestyle changes and adjustments that occur when you live with ALS.

### *Resource Guide 3*

**Changes in Thinking and Behavior in ALS**

This resource guide addresses how thinking and behavior may be affected by ALS and how these changes can impact disease course, symptom management, and decision making.

### *Resource Guide 4*

**Living with ALS: Planning and Making Decisions**

This resource guide reviews areas where careful planning and decision making will be required and will provide you with resources to help you and your family plan for the future.

### *Resource Guide 5*

**Understanding Insurance and Benefits When You Have ALS**

This resource guide provides strategies and helpful hints to better navigate health insurance and benefits. While understanding insurance and benefits may feel overwhelming, the guidelines outlined here should help simplify the process for you.

### *Resource Guide 6*

**Managing Symptoms of ALS**

This resource guide discusses a variety of symptoms that may affect you when you have ALS. As the disease progresses, various functions may become affected and it is helpful to understand potential changes so that you know what to expect and how to manage these new changes and symptoms.

## *Resource Guide 7*

### Functioning When Mobility is Affected by ALS

This resource guide covers the range of mobility issues that occur with ALS. It discusses exercises to maximize your mobility, as well as how to adapt your home and activities of daily living to help you function more effectively.

## *Resource Guide 8*

### Adjusting to Swallowing Changes and Nutritional Management in ALS

This resource guide will help you understand how swallowing is affected by ALS and what you can do to maintain nutrition for energy and strength and to keep your airway open.

## *Resource Guide 9*

### Changes in Speech and Communication Solutions

This resource guide covers how speech can be affected by ALS and explores a variety of techniques, technologies, and devices available for improving communication. By maintaining communication with others, you continue to make a significant difference in their lives, while retaining control of your own.

## *Resource Guide 10*

### Adapting to Changes in Breathing When You Have ALS

This resource guide explains how breathing is affected by ALS. Specifically, it will teach you the basics of how the lungs function, the changes that will occur, and how to prepare for the decisions that will need to be made when the lungs need maximal assistance.

## *Resource Guide 11*

### Approaching End of Life in ALS

This resource guide examines thoughts and feelings about dying and end of life. Approaching end of life is difficult and support is critical to help sort out feelings, expectations, and plans. By talking to friends, family, professionals, and planning and communicating your wishes, you can help prepare for the best possible end-of-life phase.

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**About The ALS Association**

The ALS Association is the only national non-profit organization fighting Lou Gehrig's Disease on every front. By leading the way in global research, providing assistance for people with ALS through a nationwide network of chapters, coordinating multidisciplinary care through certified clinical care centers and fostering government partnerships, The Association builds hope and enhances quality of life while aggressively searching for new treatments and a cure.

For more information about The ALS Association, visit our website at www.alsa.org.