RESEARCH AMERICA
DISCOVERY. INNOVATION. HEALTH.

If you think research is expensive, try disease.

INVESTMENT IN RESEARCH SAVES LIVES AND MONEY

Amyotrophic Lateral Sclerosis

Amyotrophic Lateral Sclerosis (ALS) is a progressive neurodegenerative disease that affects thousands of Americans. In individuals with ALS, nerve cells known as motor neurons located in the brain and spinal cord degenerate and eventually die. When motor neurons die, the brain loses the ability to control muscles, and people in later stages of the disease may become completely paralyzed.[1] There is currently no cure nor way of halting the disease, but research has advanced our understanding of what causes ALS and has opened doors for potential new treatments – and ultimately, a cure.[2]

TODAY

90%
of ALS cases are **"sporadic"** meaning the disease occurs in people at random with seemingly no inherited link.[2]

As many as
30,000
Americans may be living with ALS.[3]

Most patients die of respiratory failure within
2-5 years
of being diagnosed.[2]

COST

$250M to 1B:
The national economic cost of ALS due to direct medical care and indirect costs, such as lost productivity.[4]

$16K to $200K:
Range of annual healthcare costs for ALS on a per case basis.[4]

Research Delivers Solutions

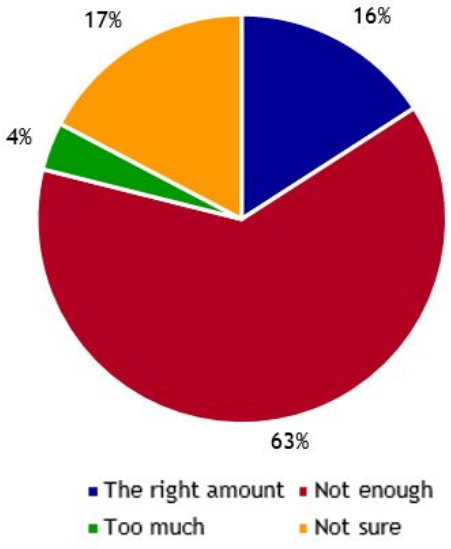
The most frequent, though not only, genetic cause of ALS appears to be a series of mutations in the ***C9orf72* gene**, which cause toxic amounts of proteins to be produced in cells. In a new study, researchers created **antibodies** which can recognize and disrupt these proteins, and will examine whether the disruption alleviates symptoms of ALS. If successful, the research may point the way toward **immunotherapy** as a possible ALS treatment.[5]

A recent study found that the abnormally high amount of certain proteins seen in ALS-affected cells interfered with the proper functioning of the ***STMN2*** gene, which helps cells **regenerate**. Researchers found that by controlling *STMN2* activity, they could **rescue damaged cells**. This discovery has strong implications for treating ALS cell damage.[6]

Advances in **stem cell research** have given scientists new ways to develop ALS treatments. Using stem cells derived from people with ALS (which have the ability to grow into specific cell types), scientists may be able to create better models of the disease, or grow healthy neuron cells for treatment.[7]

Research has shown that ALS shares some characteristics with other neuro-degenerative diseases; for example, ALS is accompanied by **neuroinflammation,** which is also common in multiple sclerosis (MS). Scientists have seen promising results using an approved MS drug, fingolimod, to treat this aspect of ALS.[8]

The U.S. spends about 5 cents of each health dollar on research to prevent, cure and treat disease and disability. Do you think that this is too much, the right amount, or not enough?



Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in January 2019

Amyotrophic Lateral Sclerosis

Then. Now. Imagine.

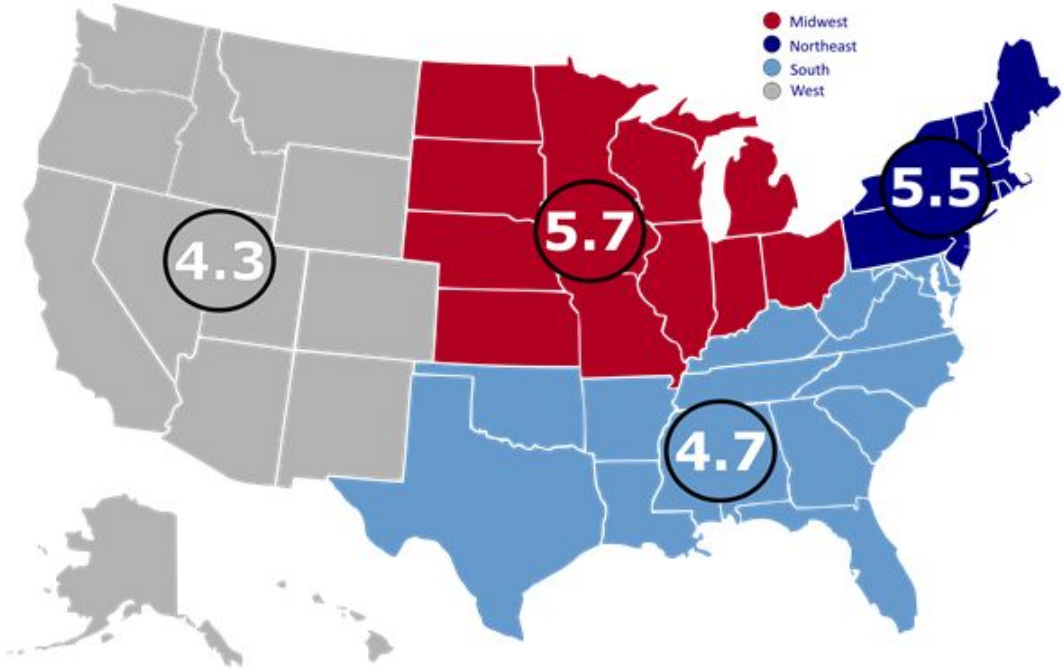
THEN
Prior to 1995, there were no medicines to treat ALS.[9]

NOW
Research has given us a better picture of shared underlying biology between some brain diseases, opening up possibilities for drugs to be used for more than one disease.[8]

IMAGINE
A cure.

The **National ALS Registry** is the single largest ALS research project ever created. Congress mandated the creation of the registry to give scientists more data about who gets ALS.[10]

Rate of ALS Deaths in 2014 by Region (per 100,000 people)



SOURCE: Centers for Disease Control and Prevention, "Prevalence of Amyotrophic Lateral Sclerosis — United States, 2014", Morbidity and Mortality Weekly Report, 2018.

1. "About ALS", The ALS Association
2. "Amyotrophic Lateral Sclerosis Fact Sheet", National INstitute of Neurological Disorders and Stroke, 2019
3. "Facts you should know", The ALS Association.
4. Santaniello. "ALS Managed Care Considerations", *The American Journal of Managed Care*, 2018.
5. Lagier-Tourenne."Testing therapeutic efficacy of human-derived antibodies targeting dipeptide-repeat proteins in C9orf72 disease", The ALS Association, 2017.
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7. Wainger et Lagier-Tourenne. "Taking on the Elephant in the Tissue Culture Room: iPSC Modeling for Sporadic ALS", *Cell Stem Cell,* 2018.
8. Berry et al. "Phase IIa trial of fingolimod for amyotrophic lateral sclerosis demonstrates acceptable acute safety and tolerability", *Muscle and Nerve*, 2017.
9. "FDA Approved Drugs", The ALS Association.
10. "The National ALS Registry", CDC, 2019.

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