

Investment in research saves lives and money



facts about:

Amyotrophic Lateral Sclerosis (ALS)

"If you think research is expensive, try disease."

- Mary Lasker 1901-1994

What is ALS?

ALS is a progressive neuromuscular disease that attacks the nerves and brains of patients affected by it. ALS patients' nerve cells gradually deteriorate, losing the ability to communicate messages to their muscles and causing a wide variety of disabilities when patients lose the ability to initiate and control voluntary movement. Most ALS patients die of respiratory failure when the muscles in their chest and diaphragm fail, usually within 2 to 5 years of the onset of symptoms. In rare cases, patients also report depression or alterations in cognitive functions involving decision-making and memory.*+^

* ALS ASSOCIATION, <WWW.ALSA.ORG>
+ NATIONAL INSTITUTE FOR NEUROLOGICAL DISEASE AND STROKE, <WWW.NINDS.NIH.GOV>
‡ MAYO CLINIC, <WWW.MAYOCLINIC.ORG>
^ MUSCULAR DYSTROPHY ASSOCIATION, <WWW.MDA.ORG>

Today:

- Approximately 5,600 Americans are diagnosed with ALS each year, or one every 90 minutes.*
- ALS occurs indiscriminately, regardless of race, ethnicity, or socioeconomic status, though it occurs slightly more frequently among men and twice as frequently among military veterans.*
- Most causes of ALS are still unknown, but we know certain gene mutations, such as SOD1, C9orf72 and others, play a role in the disease development.*
- Only one drug, Rilutek, is approved by the FDA for the treatment of ALS, though it only slows the progression of symptoms.‡

patient activist:



NAME: Michael Gollin
 AGE: 58
 CONDITION: ALS

As a boy, Michael Gollin had a strong curiosity about the wonders of nature and integrated his love for science and travel with his career as an intellectual property attorney. He has received numerous awards and accolades for his work and advocacy to improve society and make a difference, including being recognized for his contributions to the 2007 Nobel Peace Prize.

Diagnosed with ALS in 2012, Michael has not allowed the progression of the disease to keep him from being an advocate and has put his ideas to work to benefit the ALS community. He understands that people with ALS can play a direct role in the fight to find a treatment. He also knows that it's critical for government officials to hear first-hand from people living with ALS to understand the disease and why more needs to be done to advance research and support those living with ALS.

Soon after his diagnosis, Michael testified during the Food and Drug Administration's (FDA) first-ever public hearing on ALS. His message to FDA officials was direct: we need to speed the development of effective new treatments, and we need to do it now. Michael is playing an important role in a groundbreaking initiative to create an ALS drug development guidance document to be submitted to the FDA in 2016.

Michael has shared the ALS story on Capitol Hill, testified before the House Energy and Commerce Committee and provided the patient's perspective on issues such as the Dormant Therapies Act, which would remove barriers to ALS drug development and provide scientists and pharmaceutical companies with more opportunities to discover and develop treatments for ALS. For this and his other efforts, the ALS Association named Michael as the 2015 Rasmussen Advocate of the Year, the highest honor presented by the Association.

Michael truly is an inspiration. Even though ALS has robbed him of the ability to speak and walk, the disease has not slowed him down. With Michael, there is no standing still, no matter what challenges ALS presents. It's about moving forward, making progress...and making a difference.

SAVING LIVES

SAVING MONEY

HOW RESEARCH SAVES LIVES:

- Many researchers are striving to isolate the genes responsible for ALS in familial cases, and to find risk factors common to ALS patients by surveying large sets of self-reported patient data. Identifying these factors could help diagnose the disease earlier in populations at a higher risk of ALS and provide a valuable treatment window at the early signs of symptoms, when the disease is usually overlooked. These factors are especially crucial now, since no conclusive diagnosis of the disease is currently available.*

HOW RESEARCH SAVES MONEY:

- Almost half of ALS patients are disabled and unable to work, and many more work only part-time due to the disease. Innovative treatments which delay the onset of symptoms or improve the mobility of affected patients could allow these Americans to live a more active lifestyle after diagnosis and reduce or postpone their healthcare costs.*+
- Several treatment approaches under study, such as antisense, small molecule drugs and stem cell therapy show promise in the treatment of ALS to improve the quality of life.^

+ MUSCULAR DYSTROPHY ASSOCIATION, <WWW.MDA.ORG>
* CENTERS FOR DISEASE CONTROL AND PREVENTION, <WWW.CDC.GOV>
^ ALS ASSOCIATION <WWW.ALSA.ORG>

facts about: } ALS

The Cost:

❑ **PER PERSON:** \$64,000 annually and increasing. Of this, patients average almost \$20,000 annually in nonmedical costs, including necessary home renovations and professional caregiving.*

❑ **EMPLOYMENT LOSS:** In 2010, just 15% of patients with ALS were employed full-time. In contrast, 45% were listed as “disabled”.†

* LARKINDALE ET. AL., MUSCLE AND NERVE (2013) 49:3:431-438
† CENTERS FOR DISEASE CONTROL AND PREVENTION, <WWW.CDC.GOV>

Hope for the Future:

❑ **IDENTIFYING RISK FACTORS:** The Centers for Disease Control and Prevention (CDC) National ALS Registry gathers and analyzes data regarding Americans with ALS. By analyzing a large database of patient histories, researchers have been able to identify more clearly the prevalence of ALS in the U.S. The database also provides a starting point to allow researchers to search for underlying causes of the disease and recruit enrollees for new clinical trials and epidemiologic studies.*

❑ **STOPPING FAMILIAL ALS:** Studies are underway to test the effectiveness of a new drug, SOD1Rx, which suppresses all versions of the gene SOD1. SOD1 mutations are estimated to be responsible for as many as 20% of familial ALS cases, and suppressing it could slow the progression of ALS symptoms in those cases. SOD1Rx is still in the early stages of testing, but has been found safe in phase 1 clinical trials.†

* MEHTA ET. AL., MMWR, 2014/63(SS07);1-14 <WWW.CDC.GOV/MMWR>
† ALS ASSOCIATION, <WWW.ALSA.ORG>

The Ice Bucket Challenge:

In the summer of 2014, Americans from around the country participated in the ALS Ice Bucket Challenge, electing to dump ice-cold water on their heads or donate to ALS-related charities (often times both) before challenging their friends to do the same. The project directed unprecedented public attention to ALS as celebrities from Microsoft CEO Bill Gates to former President George W. Bush participated, eventually raising over \$115 million in donations. These funds have already begun funding research and scientists at Johns Hopkins University have said funds have already helped lead to a “significant breakthrough” in our understanding of ALS.*†

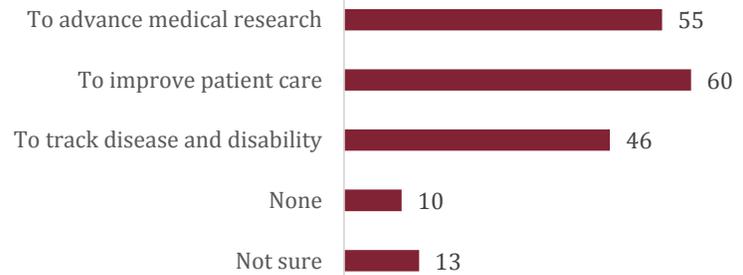
* YOUTUBE, <WWW.YOUTUBE.COM>
† ALS ASSOCIATION, <WWW.ALSA.ORG>

The Bottom Line:

ALS is a devastating disease killing and disabling Americans. It attacks the nerves of victims chosen seemingly at random, though a small portion of cases can be linked to genetic factors. We lack the ability to cure the disease or repair the damage it wreaks in the bodies of our citizens. To stop it, we must invest heavily in research aimed at identifying risk factors, curing and treating the disease, and coping with the neural degeneration it creates. The ALS Ice Bucket Challenge showed that Americans are paying attention to this disease and want to see it dealt with. It is now up to legislators, scientists, and doctors around the country to do so.

Americans willing to share personal health data for research and health care

For which of the following would you be willing to share your personal health information? (Choose all that apply)



RESEARCH!AMERICA POLL CONDUCTED IN PARTNERSHIP WITH ZOGBY ANALYTICS IN JANUARY 2015

Collaborating for Discovery:

In February of 2015, researchers from HudsonAlpha Institute for Biotechnology, Columbia University Medical Center, and Biogen announced the identification of a gene, TBK1, newly associated with ALS. This breakthrough will allow scientists to more easily diagnose ALS risk and research causes and possible therapies for the disease, but was only possible thanks to genetic data from thousands of patients, collaboration between researchers and clinicians from academia and industry, and robust funding. Industry contributions, as well as private philanthropic grants and federal research support paid for the research, including grants from the ALS Association and the National Institutes of Health. The collaboration’s researchers are now using patient-derived stem cells and mouse models with TBK1 mutations to study ALS’s mechanisms and screen new drug candidates.

* HUDSONALPHA, <WWW.HUDSONALPHA.ORG>
** CIRULLI ET AL. SCIENCE, 2015. (347)1436-1441.

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