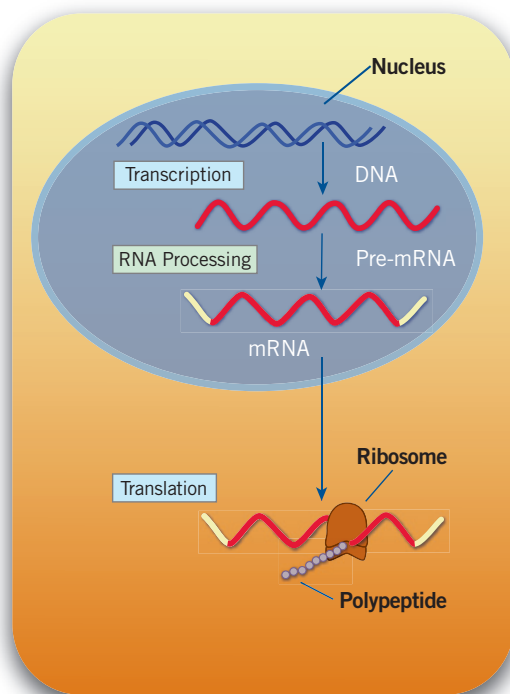


RNA Emerges as a Key Focus for Understanding ALS and Treatment Strategies

By Richard Robinson



DNA leads to the production of messenger RNA (mRNA), which is used as the instruction set for building polypeptides (proteins).

Twenty years ago, when scientists thought about RNA, if indeed they gave it much thought at all, it was mainly as a simple and well-behaved messenger, an information courier between the genes that determine heredity and the proteins that do the work of every cell. In that view, genetic diseases were due to faulty genes encoding faulty proteins, with RNA serving simply to shuttle the faulty information between them.

However, the picture that has emerged since then is anything but simple. Scientists now realize that RNA can cause disease itself, and understanding how it does so has become a central focus of a great deal of research in amyotrophic lateral sclerosis (ALS) and other neurodegenerative diseases. They also believe that in RNA may be a key target for treatment of ALS. The ALS Association has been at

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What is RNA?

Like its chemical cousin DNA, RNA (ribonucleic acid) is formed from individual building blocks, which in RNA are abbreviated as A, C, G, or U. These link together, in a sequence dictated by DNA, to form short or long molecules with a wide variety of functions. Short RNA molecules regulate when and where genes are turned on in different cells and target foreign DNA or RNA for destruction. Long RNA molecules carry instructions from the cell nucleus to the periphery to build proteins. These “messenger RNAs” are often processed, with pieces snipped out and the remainder stitched together, before they are released to do their job.

Today's Faces of Lou Gehrig's Disease

The 2014 Extended Campaign for ALS Awareness Month Emphasizes Lou Gehrig's Farewell Speech and Need to Support Research

By Stephanie Dufner

May, which is ALS Awareness Month, marks a time for the ALS community to share information with others who may not be aware of this fatal disease. The ALS Association has asked those who have lived with ALS to share their stories online to put a “face” on Lou Gehrig's Disease.

This year, The Association is commemorating the 75th anniversary of Lou Gehrig's farewell speech to baseball. Gehrig and his famous speech serve as the cornerstone of The Association's 2014 awareness campaign, which began in May and runs through July, the month the “Iron Horse” announced his retirement from baseball due to his ALS diagnosis. To many Americans, Gehrig embodies the first face of ALS, a disease virtually unheard of in 1939. This year's story submissions highlight today's faces of ALS. Tales of mothers, fathers, grandparents, spouses and friends tell of the resolve, resilience and bravery these individuals have exhibited in confronting the disease, traits that Gehrig also displayed during his two-year battle with ALS.

Visitors to the microsite, www.alsa.org/75, who read these stories also have the option to share them on Facebook and Twitter. They can spread additional ALS awareness during the three-month

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A New Beginning is Not Starting Over

Message from The ALS Association President and CEO Barbara J. Newhouse

ALS has a history, which is far too long from my perspective. It was first described in 1869 and then brought to national and international attention in 1939 with Lou Gehrig’s public announcement that he had a disease that would take his life and his name. Everyone touched by ALS agrees that it’s “high time” we put an end to this dreadful disease.

Easier said than done? Perhaps, but The ALS Association is determined to lead the way to truly effective treatments to slow and halt the disease progression as quickly as possible. Recent discoveries indicate, for the first time in history, that more and more we are understanding how ALS works on a molecular level. With this understanding, we are in striking distance of learning how to intervene in cell processes to interrupt neuronal destruction and/or protect neurons from harm.

Through national and international collaborations, our global research program is fostering partnerships across the boundaries of academia, industry, government and other non-profit organizations. Our national care network includes chapters, centers and clinics providing unparalleled, multidisciplinary support to those with ALS and their families. The Association’s public policy efforts are winning support for ALS-related issues and government research funding. In addition, The Association is the recognized source for vital information about the disease and important initiatives in the ALS community.

Having recently joined The ALS Association, I am inspired by what I see happening both within the organization and in the broader public arena. Publicly, I thank Jane Gilbert for the leadership she has provided and the progress that has been made during the past five years. She has given guidance, motivation and compassion to those around her.

I am truly honored and excited to be given the opportunity to lead The ALS Association and look forward to working with every one of you to help write the final chapter of the history of Lou Gehrig’s Disease. With your continued support of The Association, I know, in my heart, we can do it.



President and CEO
Barbara J. Newhouse

RNA Emerges as a Key Focus

Continued from page 1

the forefront of research to understand the role of RNA in disease and develop treatment strategies that target RNA in ALS.

RNA-related Genes in ALS

Two key advances turned the research community’s attention toward RNA in ALS. The first was the discovery of ALS-causing genes TDP-43 and FUS, both of which, in their normal role, bind to RNA and help process it. This suggested that the disease might involve a defect in RNA processing.

The second was the discovery of the C9orf72 gene. Mutations in this gene, whose normal function is still unknown, cause up to 40 percent of all familial ALS and about six percent of sporadic ALS. The mutation is an expansion of a short section of the gene composed of six repeated letters. In the mutated gene, that section grows to hundreds or thousands of repeated units. The messenger RNA formed from the gene also contains that long repeated section.

This long RNA is “sticky” and forms clumps within the cell that may contribute to the ALS

disease process through several mechanisms. The clumps are believed to trap other essential cell molecules, including ones that help maintain motor neurons or control other genes. Loss of these molecules may cause or accelerate death of the neurons. The long RNA also causes production of unusual protein-like molecules not seen in other cells (called “RAN peptides”), and these may cause problems as well.

Antisense Against RNA

ALS Association-supported scientists are at the forefront of developing therapies that make use of these new discoveries. One of the most promising approaches uses “antisense” molecules against the RNA molecules that cause the problems. An antisense molecule is created as a matching opposite (“anti”) to the disease-causing RNA and binds it specifically. When that occurs, cellular defense machinery destroys the target RNA, leaving the antisense molecule to bind to more of the target RNA. Antisense therapy has been shown to be safe in people with ALS caused by the SOD1 gene. Clinical trials using antisense against C9orf72 are currently in the development stages.

VISION

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The year 2014 recognizes a milestone in baseball history—the 75th anniversary of Lou Gehrig’s farewell speech. In May, which is National ALS Awareness Month, The ALS Association debuted a three-month campaign emphasizing

Gehrig’s final appearance in Yankee Stadium, where he announced his retirement from America’s favorite pastime due to his ALS diagnosis.

The campaign, primarily highlighted on the microsite www.alsa.org/75, runs through July, the month Gehrig told his legions of fans on Independence Day 1939 that he “...might have been given a bad break, but I’ve got an awful lot to live for.” It aims not only to commemorate Gehrig’s goodbye to baseball but also to bring awareness of the disease to those who are unfamiliar with it. This microsite contains several elements, including links to Gehrig’s ALS story, print public service announcements and downloadable social media assets, as well as articles discussing recent scientific discoveries pertinent to ALS.

“Visitors to the microsite can read informational links about ALS. The microsite also puts a ‘face’ on the disease that bears Lou Gehrig’s name,” said Carrie Martin Munk, The ALS Association’s Chief Communications and Marketing Officer. “Gehrig’s story is compelling in itself. He was admired both on and off the field, and ALS impacted him in the prime of his life, just like it does to many of the individuals battling it today.”

The Association invites those most familiar with Lou Gehrig’s Disease to tell their stories and submit a photo or brief video of themselves or their loved ones with ALS. Visitors to these stories can share these tales on social media and have the option of supporting ALS research with a donation.

“The 75th anniversary of Lou Gehrig’s farewell speech underscores the urgency that exists to fund research to find the causes, treatments and a cure for this deadly disease,” added Munk.

A Change for Good

75th Anniversary of Lou Gehrig’s Farewell Speech Serves as Inspiration to Name Cure for Lou Gehrig Instead of a Disease

By Stephanie Dufner

This multi-faceted campaign also features elements independent of the microsite. A full-page version of the print PSA recently appeared in an issue of *Time* magazine, which has an estimated readership of more than 3.2 million. The periodical donated the ad space that contained an image of Gehrig standing with his arms crossed and head bowed before a packed stadium crowd.

A minute-long television PSA showcasing Gehrig’s career and discussing the need to cure ALS is also slated to air on the History® channel and A&E Networks this summer. History, a corporate partner for the campaign, donated the broadcast air time. Major League ballparks will also show this PSA on Jumbotrons in stadiums across the United States on July 4 to recognize the anniversary of Gehrig’s speech.

The Association additionally distributed a multimedia news release to more than 300 online news outlets focusing on the campaign’s goals and featuring the campaign’s corporate partners, History, CytoKinetics and Pride Mobility/Quantum Rehab. During May, Pride Mobility matched donations to ALS research up to \$75,000 and donated 10 lift chairs to The Association’s chapters for people with ALS. CytoKinetics will match donations in July.

“The 75th anniversary of Lou Gehrig’s farewell speech underscores the urgency that exists to fund research...”

—Carrie Martin Munk
Chief Communications and Marketing Officer
The ALS Association



Every Dollar Makes a Difference

Turn your summer barbecue or lemonade stand into a fundraiser to support The ALS Association! **One Dollar Difference** is the perfect solution to turn any event you're hosting into an opportunity to find the cure for ALS. And getting started is fun and easy!

First, visit www.one-dollar-difference.org to register your event online. Then, customize your individual event webpage with the details of your event and motivation for supporting The Association; upload personal photos and video; import your email address book to send messages to attendees and supporters to keep them updated; and log into your One Dollar Difference Fundraising Center often to keep track of your progress.

4. The One Dollar Difference online fundraising program supports the mission of The ALS Association, bringing us closer to realizing our vision to create a world without ALS.

To make an impact in the fight against ALS, please visit www.one-dollar-difference.org today!



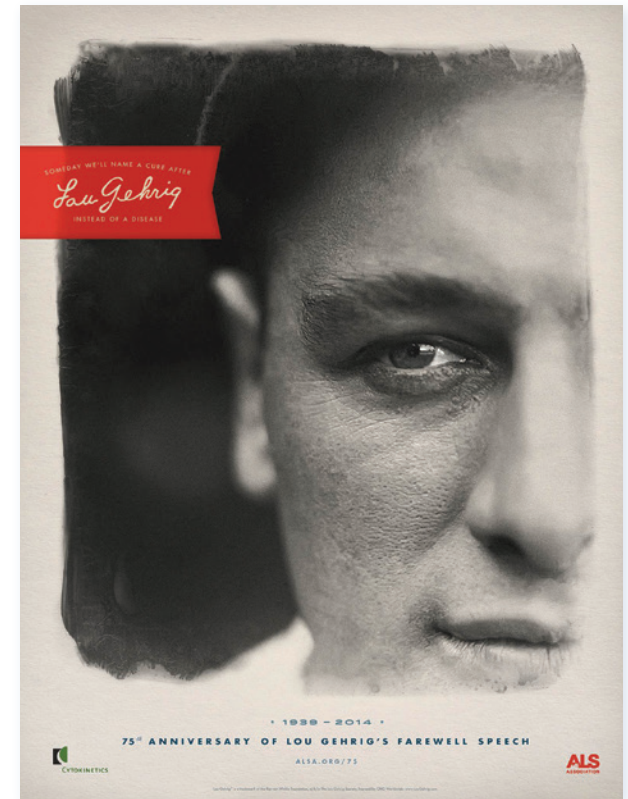
Today's Faces

Continued from page 1

campaign by downloading social media graphics to place on their Twitter or Facebook pages, YouTube channel, or personal or organizational website.

Another component of this year's campaign is the emphasis on the acceleration of research that has transpired since Gehrig received his ALS diagnosis. As presented in the infographic on page 3, scientists specializing in the disease had found only one gene linked to ALS from 1939 to 2003. In the past decade, a multitude of ALS-related genetic discoveries have occurred: Researchers have discovered 20 genes connected to the disease. Some of these findings were funded through The ALS Association's TREAT ALS™ research program.

To underscore the critical need to support research into the disease, site visitors have the option of donating during the campaign's duration. Each story's page features a "Donate" button, which indicates that 100 percent of contributions to the campaign will go toward ALS research and therapy development.



Ad commemorating 75th anniversary of Lou Gehrig's farewell speech, which appeared in *Time* magazine

Honoring Lou's Legacy While Spreading ALS Awareness

For the past several years, The ALS Association and its nationwide network of chapters have partnered with Major and Minor League Baseball teams to spread ALS awareness. In honor of the 75th anniversary of Lou Gehrig's farewell speech to baseball, the History Channel collaborated with The ALS Association to create a public service announcement (PSA) to remember Gehrig

and his contributions to America's favorite pastime. The PSA, which is slated to air on television and at Major and Minor League ballparks across the United States this summer, also emphasizes the need to fund research, which will find the causes, treatments and a cure for the disease that continues to affect tens of thousands of American families.

Growing and Enhancing Our Certified Treatment Centers of ExcellenceSM

By Katie Sweeney

Over the past year, The ALS Association has been expanding and enhancing our Certified Treatment Centers of ExcellenceSM program by recertifying existing centers and adding several new provider organizations to our nationwide network.

The program, originally launched in 1998, recognizes clinics that provide evidence-based, multidisciplinary ALS care and services in a supportive atmosphere—emphasizing hope and quality of life. Each clinic must meet strict criteria and follow the American Academy of Neurology (AAN) ALS Practice Parameters.

“Our Certified Treatment Centers of Excellence advance and support the gold standard of care in ALS,” said ALS Association Chief Care Services Officer Kimberly Maginnis. “By adding new centers and strengthening the program, we’re working to ensure that more patients can receive the comprehensive care, support and information they need to cope with this disease.”

Clinics undergo a rigorous process to achieve certification and recertification. Criteria include:

- Demonstrated knowledge and experience with ALS patients
- Active involvement in ALS-specific research
- On-site, multidisciplinary team, including a neurologist
- Availability of neurological diagnostics/imaging
- Implementation of AAN ALS Practice Parameters
- Active relationship with an ALS Association chapter

The Association reviewed and revised the program in 2012, receiving input from ALS Association staff, researchers and clinic medical directors. The recertification process began last year.

“The key to this program is the close collaboration between our national office, local chapters and the clinics,” explained Certified Center Programs Director Nicole Yarab, R.N., B.A. “Certification is not just a moment in time; it’s an ongoing commitment to working together to ensure the best possible care for people with ALS.”

To find a Certified Treatment Center of Excellence near you, visit:
www.alsa.org/community/certified-centers



Creating Your Own Community of Hope

Looking for a way to recognize a special someone by creating a lasting legacy in his or her name? The ALS Association's Community of Hope program offers the opportunity to establish an online fund to honor or memorialize your loved one and their fight against ALS.

Not a web guru? No problem. We're here to help!

Earlier this year, we upgraded the design and features of the Community of Hope. Now, registering your fund on our new and improved website is easier than ever, and we offer online resources and staff support to help you along the way.

When you're ready to get started, you will be able to personalize your individual fund web page by writing your story, uploading photos and video, setting a fundraising goal, enabling your own Community of Hope blog, and sending emails to family and friends to ask for their support.

Proceeds raised through the Community of Hope support local patient care and family services, national public policy initiatives, and a global ALS research program, all while promoting the hope of a world without ALS to all of those affected by the disease.

To learn more and establish your Community of Hope tribute fund today, please visit www.community-hope.org.



AN EDUCATIONAL VIDEO SERIES

Respiratory Decisions in ALS

By Cynthia Knoche

A diagnosis of ALS presents a person and their family with long sought-after answers to their progressive symptoms. Along with receiving a diagnosis of a progressive neurodegenerative disease, comes the challenge of working with their healthcare team to develop a strategic healthcare plan as ALS symptoms unfold. Decisions related to use of mobility and communication devices are generally a direct response to changing functional needs. Decisions regarding respiratory support can be more challenging as many people are unfamiliar with the variety of respiratory support options available.

Regardless of where the initial weakness occurs, eventually the muscles that control breathing become weak. "Respiratory Decisions in ALS" is a three-part video series that discusses how ALS affects the respiratory system; what types of techniques, tools and equipment are available to address the increased work of breathing that goes along with increased respiratory muscle weakness; and what full-time mechanical ventilation options are available. Produced by the The ALS Association in collaboration with the Forbes Norris MDA/ALS Research Center and the Les Turner ALS Foundation, each 15 to 18 minute segment highlights information shared by

ALS experts—physicians, nurses, and a respiratory therapist—and perhaps more important, by those individuals diagnosed with ALS and their families.

A common theme throughout the three-part series is the realization that respiratory challenges are part of ALS, and the very best way to address these challenges is to learn about available options and actively participate in developing a strategic healthcare plan with a personal healthcare team. The types of respiratory support a person chooses to include in their plan are based on their own beliefs, as well as their family and financial situations. Some people make changes to their plan as events unfold.

The critical point is that people should participate in developing their own care plan so choices are not being made for that person in an emergency situation. These educational videos are available to

view through ALS Association Certified Treatment Centers of Excellence and ALS Association chapters as well as by visiting The ALS Association national website, www.alsa.org.

Support for the production of the "Respiratory Decisions in ALS" educational video series was made available by Sanofi US.



Don't Worry! You Don't Need a Will

Want to make the government love you?

We've got a surefire way you can make Uncle Sam one cheerful fellow, and all you have to do is...nothing. Because by doing nothing you give the government more power and more of your money. In return, Uncle Sam becomes just like one of the family, and as such, becomes your happiest heir!

Just follow these simple steps:

- 1. *Don't write your will.*** Without a will the court will get to decide how to disburse your assets. Of course, the court doesn't know you have stepchildren you're fond of or that you want to remember a few of your favorite charities in your will—and the court doesn't care.
- 2. *Don't write your will.*** Without a will the court will select someone to handle your estate during probate. Even if you would have selected a close friend to have this responsibility, the court won't know that and won't care.
- 3. *Don't write your will.*** If you have minor children for whom guardianship is important, guess what, the court will pick the guardian for you. This means you can sit back and relax—the court will decide who should care for your kids.

The Government Will Take Care of It for You



- 4. *Don't write your will.*** The estate tax dollars that your government could collect will be used for, well, anything the government chooses. Just think what a few of your favorite charities—those you've been supporting during your life—could do with that money.
- 5. *Don't write your will.*** Your heirs will be grieving after you are gone. Why not saddle them with the added responsibility of figuring out your affairs and what decisions you might have made; because now they will just have to guess?

Pardon the irony, but have we made our point? These are some **darn good reasons** why you should write your will. We at The ALS Association urge you not to subscribe to the idea that you, your family and the charities you care about are better off if you die without a will. Don't continue to put off the minor inconvenience of making a will. The peace of mind will be worth every minute of time you spend on getting your will done.

Let Uncle Sam take care of himself. Your family will be thankful. Your favorite charities will be thankful.

By the way, we've heard on more than one occasion that some of you are a little nervous about meeting with an attorney. But an attorney who specializes in wills and estate planning is the right person to seek out because he or she can help you accomplish your goals. We can also send you some information on writing a will and, if you like, ways you can include The ALS Association and your other favorite charities in your plans.

Writing a will is too important to delay. It's important for you, for your heirs, and for The ALS Association. Planned Gifts enable you to leave an extraordinary legacy and make a transformational difference.

Walk to Defeat ALS® Brings Hope to Thousands Living with ALS

By Heather McDonald

The Walk to Defeat ALS® began in 2000 as a small group of committed people joining together to walk in honor of their loved ones suffering from this devastating disease. Since that time, the Walk to Defeat ALS program has grown into a nationwide program.

Each year approximately 175,000 participate in more than 165 walks across the country to raise money to fight ALS. To date, the Walk program has raised in excess of \$182 million. Monies from the Walks go toward local care services programs, research to find treatments and a cure for the disease, and federal, state and local public policy efforts.

The Walk to Defeat ALS is about much more than simply fundraising. It raises awareness about Lou Gehrig's Disease and brings together those with ALS, their families and others that are in some way touched by the disease, uniting them to realize that they are not alone in their fight against ALS.

8. *"My hope now is that by raising awareness and participating in the Walk to Defeat ALS we can help make a difference so that someday others will not suffer as my father did and other families will not have to say goodbye to their loved ones much too soon."*—Cynthia K. from Hiram, Ohio

This year's Walk to Defeat ALS fundraising goal is \$25 million. Join us in the fight against ALS and participate in the Walk by visiting www.walktodefeatal.org for more information.

175,000
Participants

FY14 - \$23.5 Million



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165
Walks

