

# Tofersen and the FDA Approval Process

## **JEREMY HOLDEN**

Director of Mission Communications  
The ALS Association

## **NEIL THAKUR, PhD**

Chief Mission Officer  
The ALS Association

## **TIMOTHY MILLER, MD, PhD**

Professor of Neurology  
Washington University in St. Louis

## **RICH BRENNAN, M.A.**

Vice President, Federal Affairs  
The ALS Association





**Making ALS **livable** for  
everyone, everywhere,  
**until we can cure it****

## What **Livable** Means

People with ALS will have a fundamentally different experience than they have today.

- Longer lives
- Improved quality of life
- Preventing loved ones from getting ALS



- **Optimize Current Treatments and Care**
- **Prevent or Delay the Harms Associated with ALS**
- **Find New Treatments and Cures**



- **Optimize Current Treatments and Care**
  - Support over 20,000 people with ALS and their families
  - Certify over 90 multidisciplinary treatment centers
  - Effective care is affordable and accessible
- **Prevent or Delay the Harms Associated with ALS**
- **Find New Treatments and Cures**



- **Optimize Current Treatments and Care**
- **Prevent or Delay the Harms Associated with ALS**
  - Launched first prevention RFA in 2022
  - Supporting genetic testing and fighting genetic discrimination
  - Preventing financial harms: in 2022, over \$600,000 in savings and benefits for families with ALS using our PAF helpline
- **Find New Treatments and Cures**





- **Optimize Current Treatments and Care**
- **Prevent or Delay the Harms Associated with ALS**
- **Find New Treatments and Cures**
  - Relentless focus on speed and impact for ALS research
  - Current funding \$53 million in research around the world, ~40 potential treatments
  - New Treatments Approved





**TIMOTHY MILLER, MD, PhD**  
Professor of Neurology  
Washington University in St. Louis  
<https://ALScenter.wustl.edu/>





# The Association Supports the Approval of Tofersen

- The Association support since at least 2004 over multiple grants
- We do not take a position on FDA approval for any drug until we conduct a review with independent experts



# The Association Supports the Approval of Tofersen

- Tofersen failed its primary endpoints, and it appears the trial was not long enough to detect a clinical change
- Tofersen has a clear biological effect after 3-4 months of treatment, with clinical change occurring months later
- After a year of study, people who got the drug six months earlier do appear to be declining at a much slower rate



# The Association Supports the Approval of Tofersen

We recommend accelerated approval because

- Strong biological effect
- Link between the biological effect and clinical effect
- The devastating nature of ALS and the challenges of ultra rare research
- An ongoing study, expected to complete in 2027, is the fastest way to meet the requirements for accelerated approval

<https://www.als.org/research/emerging-drugs/tofersen>



# TAKE ACTION NOW





# Comments to the FDA

Submit comments to the FDA at

<https://www.regulations.gov>

Search docket FDA-2022-N-0691

Or use this QR Code  
for instructions



<https://www.als.org/stories-news/als-association-submits-public-comments-fda-review-tofersen>

Deadline to submit your comments:

**March 21, 2023, before 11:59pm ET**



# Join Our Upcoming Webinar

## REGISTER TODAY!

Join us for our ALS Research  
Appropriations Webinar March 21st, 2023

12:00-2:00 PM EDT

Register at [ALS.org/ALSResearchFunding](https://ALS.org/ALSResearchFunding)



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# Get Alerts

## SIGN UP FOR ADVOCACY TEXT ALERTS!

Text "ALS" to  
855-469-2621



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## Talk to Your Doctor

- Genetic testing needs to be ordered by a health care professional. You can also for testing or a referral to a genetic counselor.

## ALS Identified

- This program – sponsored by Biogen and offered through the diagnostic company Invitae – provides free genetic testing and post-test counseling to people with ALS and their families.



[www.als.org/genetictesting](http://www.als.org/genetictesting)

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For more information:  
email us at [feedback@als.org](mailto:feedback@als.org)

