

Thank you for joining the **Leap Day Special** webinar!  
We are admitting audience members from the waiting room.  
**Please allow a few moments for the webinar to begin.**



# HEALEY ALS Platform Trial

Weekly Q&A – February 29, 2024



## Healey & AMG Center

Sean M. Healey & AMG Center for ALS  
at Massachusetts General Hospital



Calico



The AMG Foundation



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The last leap day was February 29, 2020. As we reflect on progress over the last four years, we are immensely grateful to trial participants and everyone in the ALS community who has supported research in many different ways.

**Progress in ALS research and advances in clinical care options would not be possible without your partnership**

*Thank You*

# ALS Therapy Options Are Expanding

- **Disease Modifying Medications - 4 approved drugs in the US**
  - \***Riluzole** (1995)
  - \***Radicava** (edaravone; 2017-IV; May 2022-oral)
  - \***Relyvrio** (PB and TURSO; September 2022)
  - \***Tofersen, SOD1** (April 2023)- first ALS approval under the accelerated approval pathway
- Medications and supportive care to treat symptoms

 **Early diagnosis and access to care are paramount**

# Short-Term and Long-Term Goals:

- **Slow/Stop ALS**

- Likely to require a cocktail of products
- Targeted drugs, if applicable

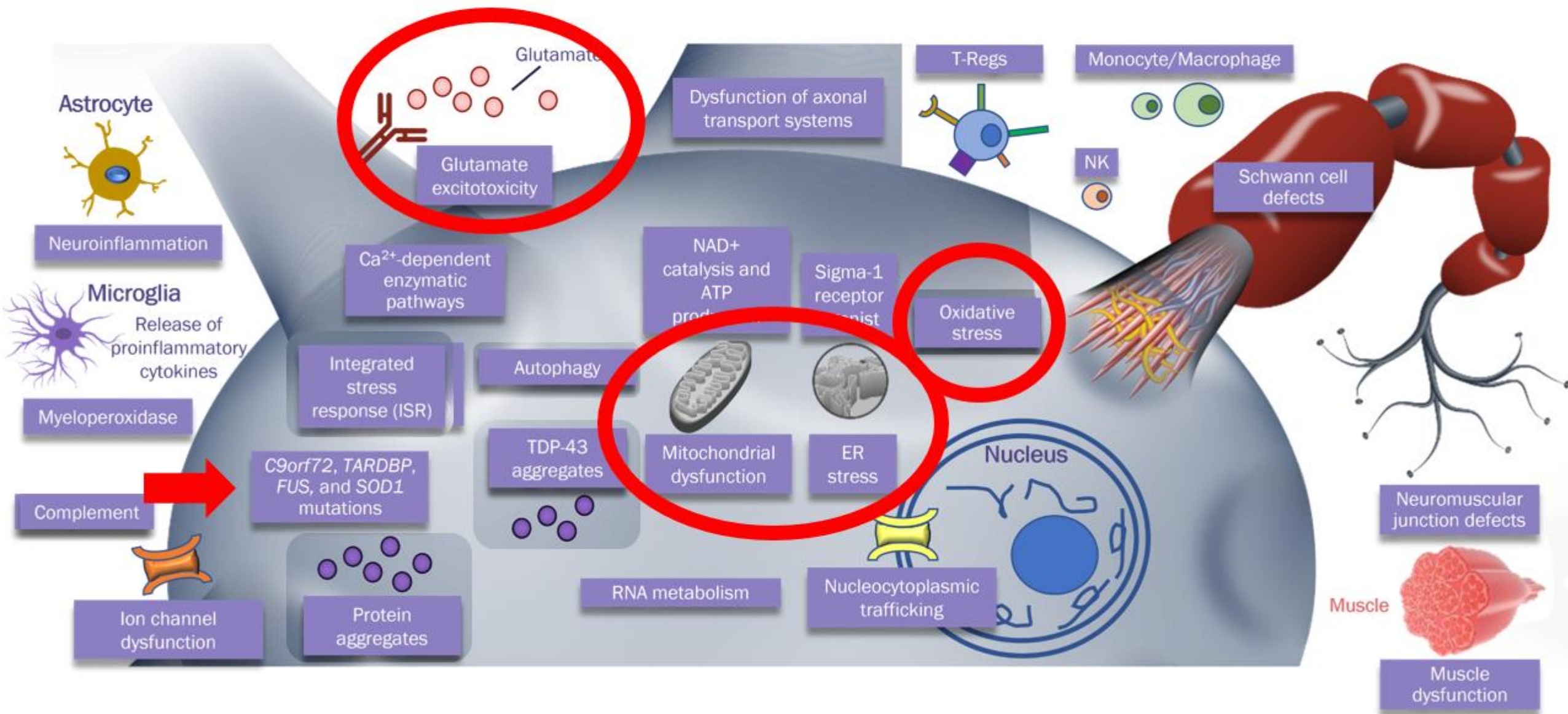
- **Prevent ALS**

- **Gene carriers**

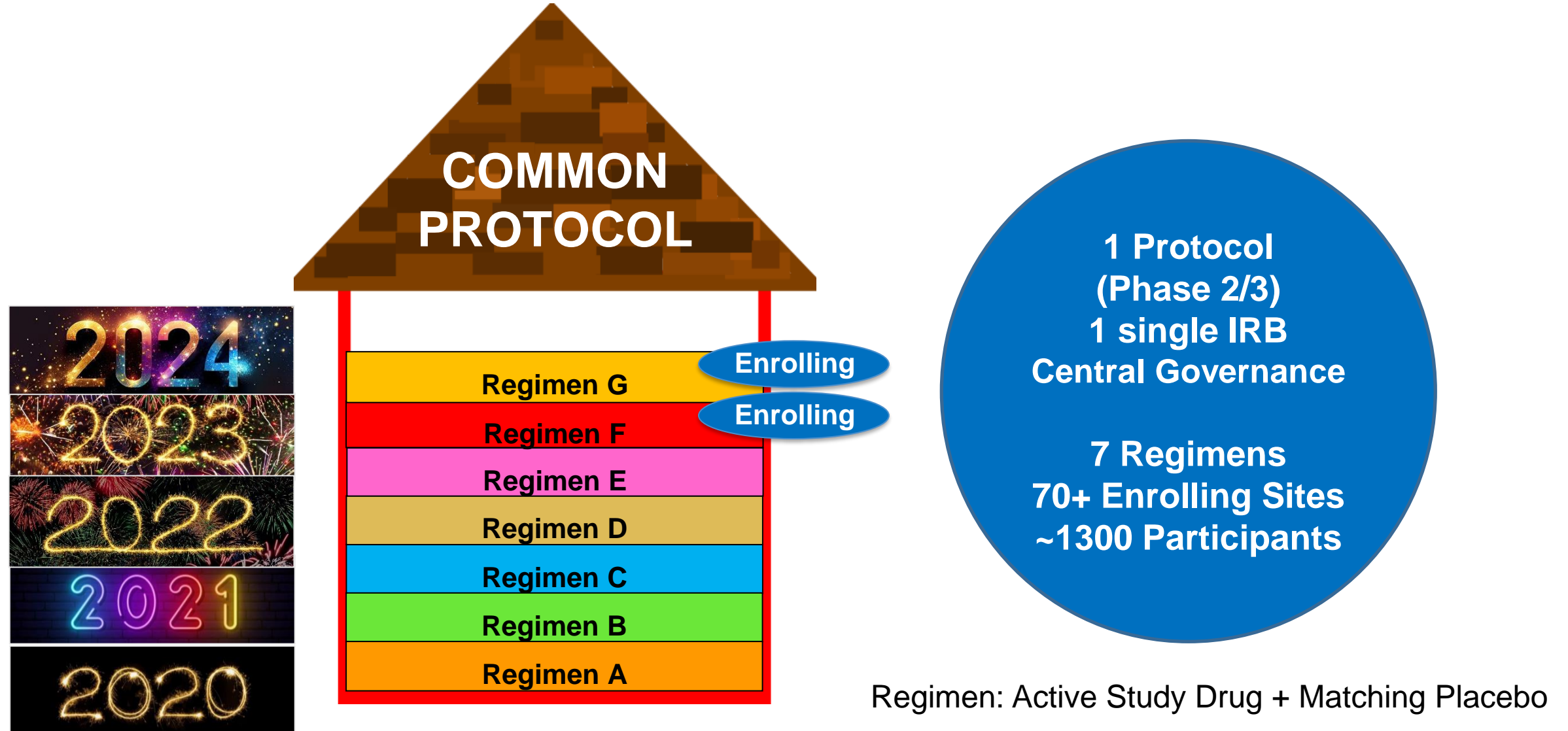
- **Reverse ALS**

- > 250 companies in ALS space
- Active clinical trial networks
- Engaged patient population
- Engaged, collaborative foundations

# Multiple mechanisms are implicated in ALS



# Common protocol and shared infrastructure allow for operational and scientific efficiencies





A- Zilucoplan



B- Verdiperstat



C- CNM-Au8



D- Pridopidine



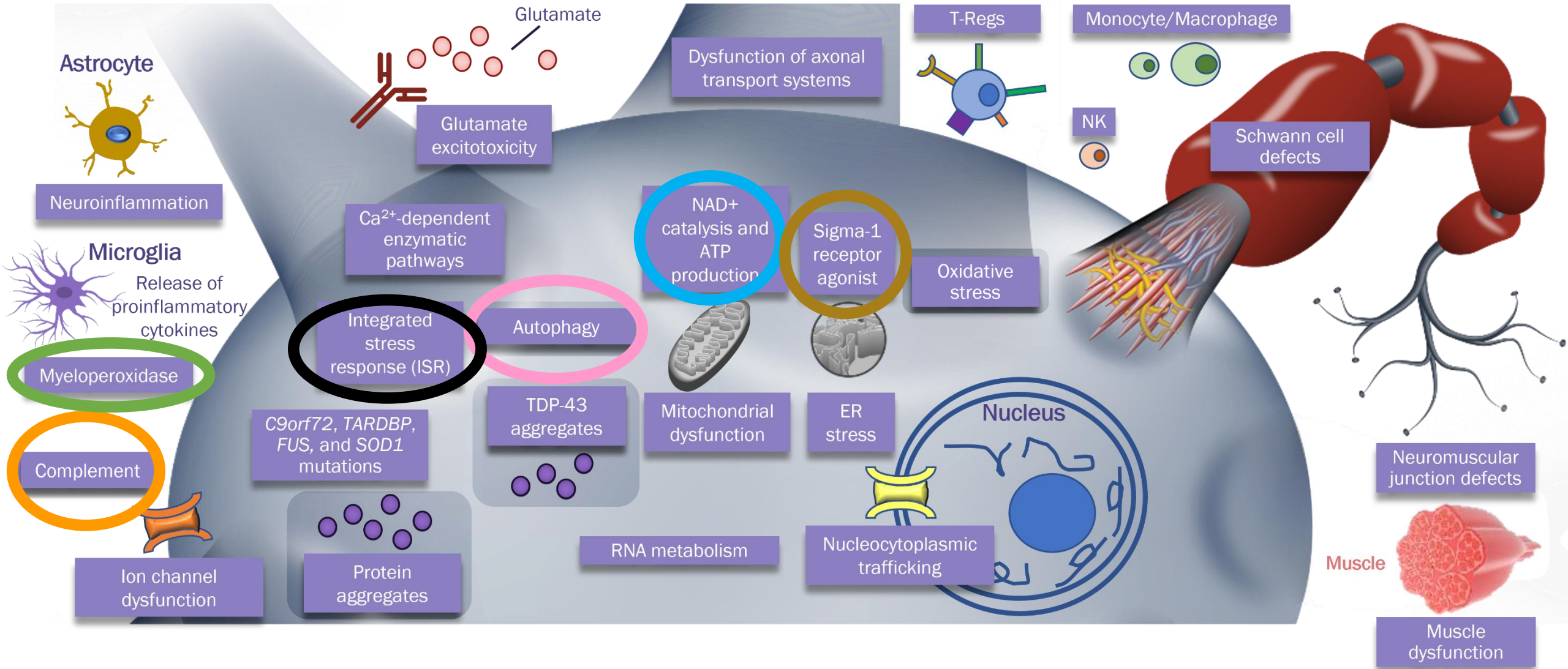
E- Trehalose



F- ABBV-CLS-7262



G- DNL343





# Platform trials are a unique opportunity to advance science



**DNA** – whole genome sequencing



**Neurofilaments** –for all regimens



**Biomarkers** (Blood, Urine, CSF) – several drug-specific biomarkers



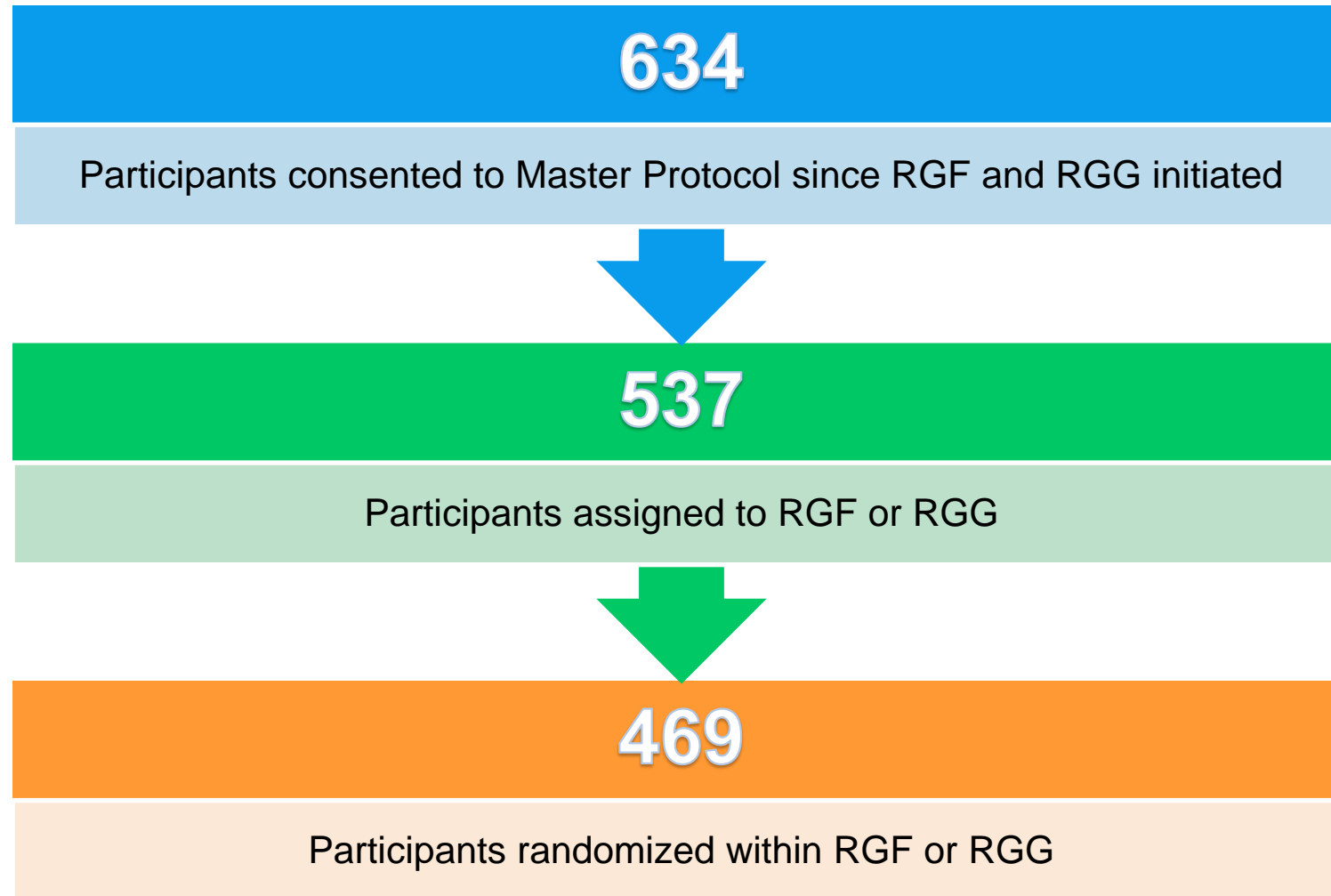
**Speech Analysis** – emerging digital biomarker



**Home Spirometry** – critical during the pandemic

**Additional biomarkers/outcome measures for upcoming regimens  
(new patient-reported outcomes; PBMCs for stem cell generation)**

# Enrollment Update: Regimen F and Regimen G

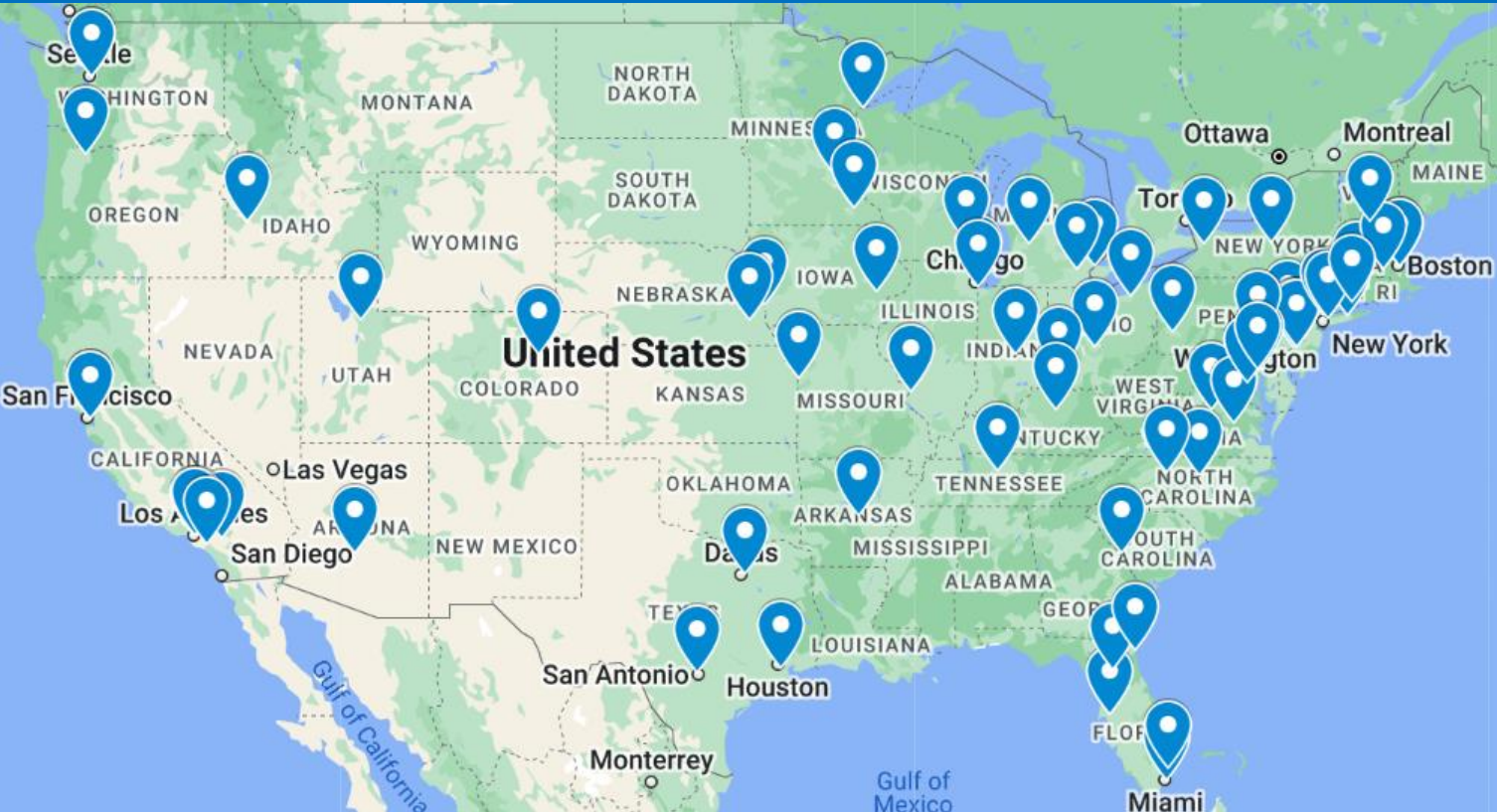


*Thank You*

for your partnership in ALS research

(as of 2/29/24)

# Providing research access across a diverse network of 70+ NEALS sites



Contact a study team near you to discuss enrollment opportunities



<https://bit.ly/3g2NZr5>

# Sharing our experience

*Meetings with disease-specific networks both in the US and globally*



## Disease Areas

1. ALS
2. Alzheimer Disease
3. Duchenne Muscular Dystrophy
4. FSHD
5. Myotonic Dystrophy
6. Frontotemporal Dementia
7. Parkinson Disease
8. Progressive Supranuclear Palsy (PSP)
9. Traumatic Brain Injury
10. Spinal Cord Injury
11. Vanishing White Matter Disease
12. Depression
13. Neurofibromatosis (NF)
14. Scleroderma
15. Idiopathic Pulmonary Fibrosis
16. Fibrodysplasia Ossificans Progressiva (FOP)
17. Vascular Malformations

**New Regimen  
Application Form:**



**Master Protocol, Publications, and Other Documents Available at:**  
<https://www.massgeneral.org/neurology/als/research/research-partners>

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[spaganoni@mgh.harvard.edu](mailto:spaganoni@mgh.harvard.edu)

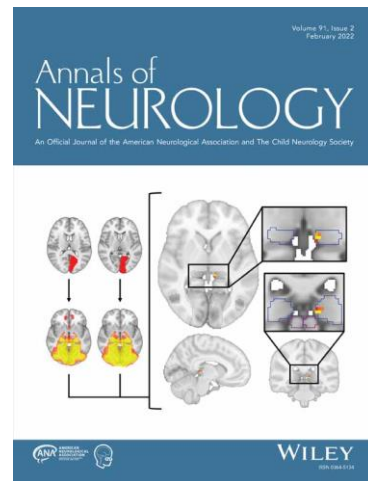
# Sharing our experience

*Research publications available via open access*

## Adaptive Platform Trials to Transform Amyotrophic Lateral Sclerosis Therapy Development

First published: 21 December 2021

Open Access

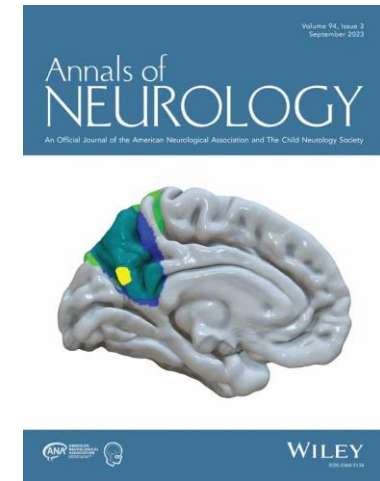


<https://onlinelibrary.wiley.com/doi/10.1002/ana.26285>

## Design and Statistical Innovations in a Platform Trial for Amyotrophic Lateral Sclerosis

First published: 28 May 2023

Open Access



<https://onlinelibrary.wiley.com/doi/10.1002/ana.26714>

# Patient Navigation

## Central resource for people living with ALS



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Weekly webinar  
registration:



<https://bit.ly/3r6Nd2L>

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>

### Upcoming Webinars:

**March 7<sup>th</sup>- Weekly Q&A**

**March 14<sup>th</sup>- EAP Discussion with Dr. Jinsy Andrews (Columbia University)**

**March 21<sup>st</sup>- Weekly Q&A**



Allison Bulat