

Thank you for joining the weekly webinar!

We are admitting audience members from the waiting room.

Please allow a few moments for the webinar to begin.



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



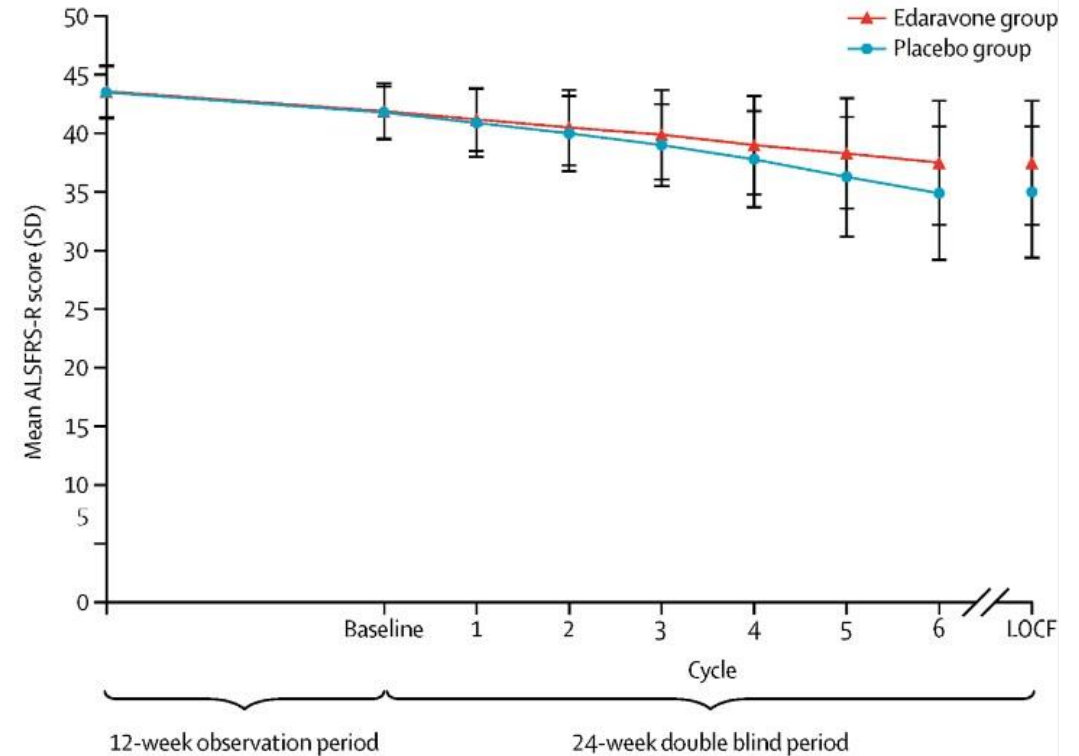
Early diagnosis and access to care are paramount

Riluzole (1995)

- Study 1 (N = 155; Bensimon et al., 1994)
- Study 2 (N = 959; Lacomblez et al., 1996)
 - Study duration: 12 to 18 months
 - Primary outcome: survival
 - Results: survival difference between active and placebo

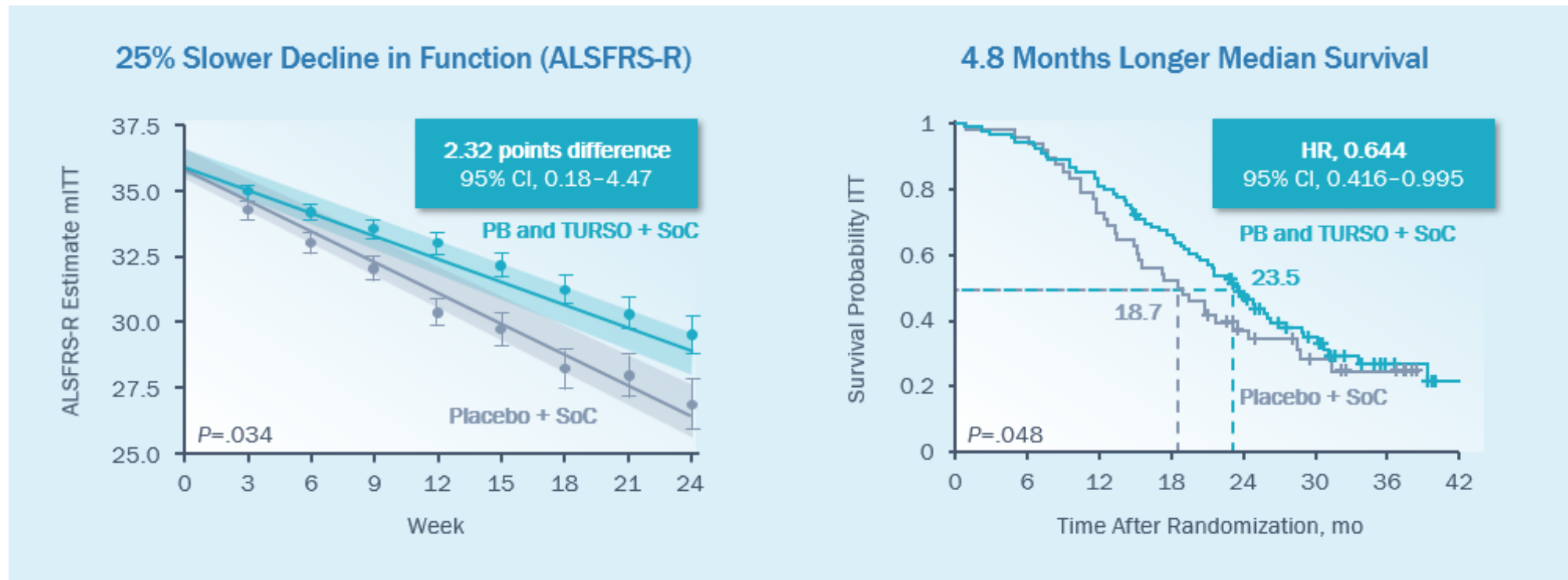
Radicava (edaravone; 2017-IV; May 2022-oral)

- Study 19 (N = 137; Abe et al., 2017)
 - Study duration: 6 months
 - Primary outcome: change in ALSFRS-R score
 - Results: difference between groups at week 24 was 2.5 points in favor of edaravone

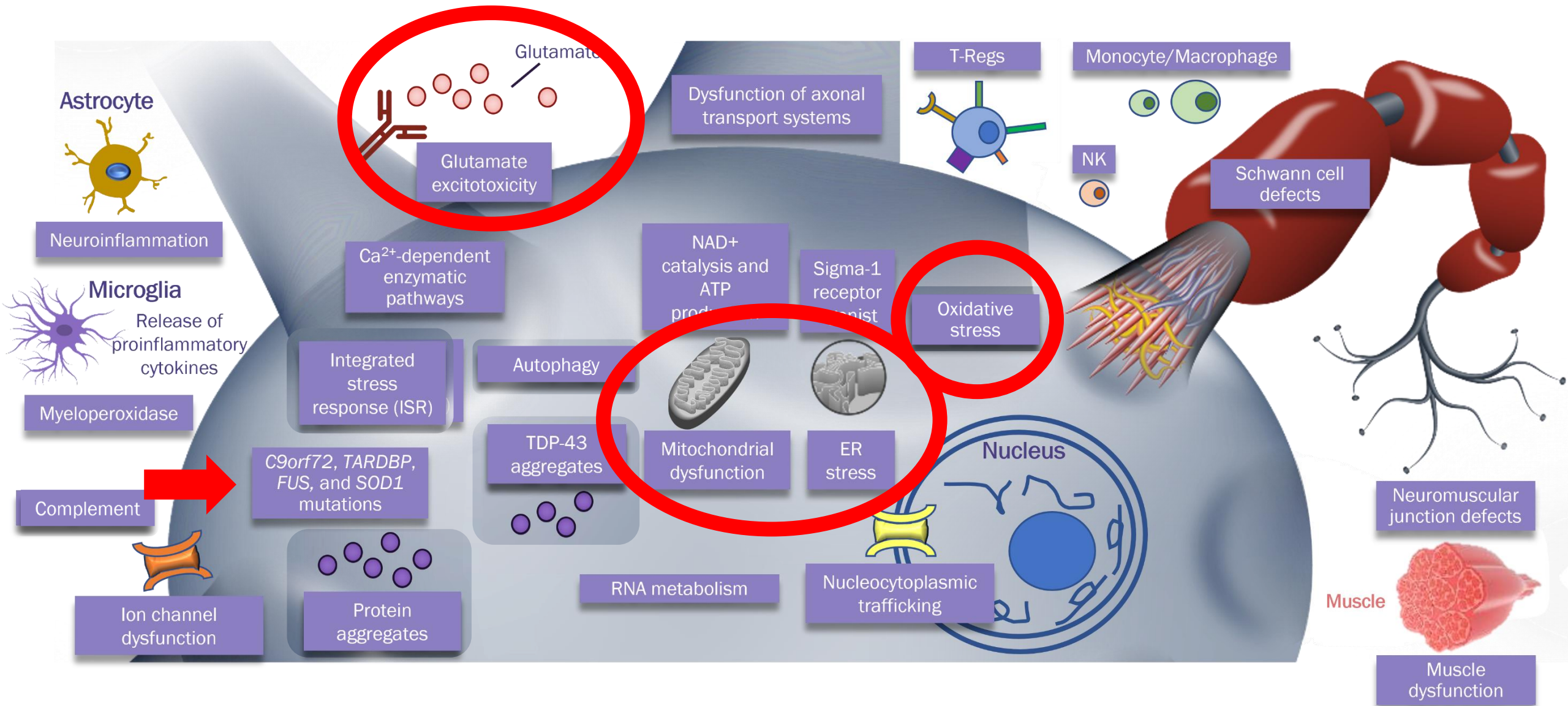


Relyvrio (PB and TURSO; September 2022)

- CENTAUR study (N = 137; Paganoni et al., 2020; Paganoni et al., 2022)
 - Study duration: 6 months



Multiple mechanisms are implicated in ALS



Short-Term and Long-Term Goals:

- **Slow/Stop ALS**

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

- **Prevent ALS**

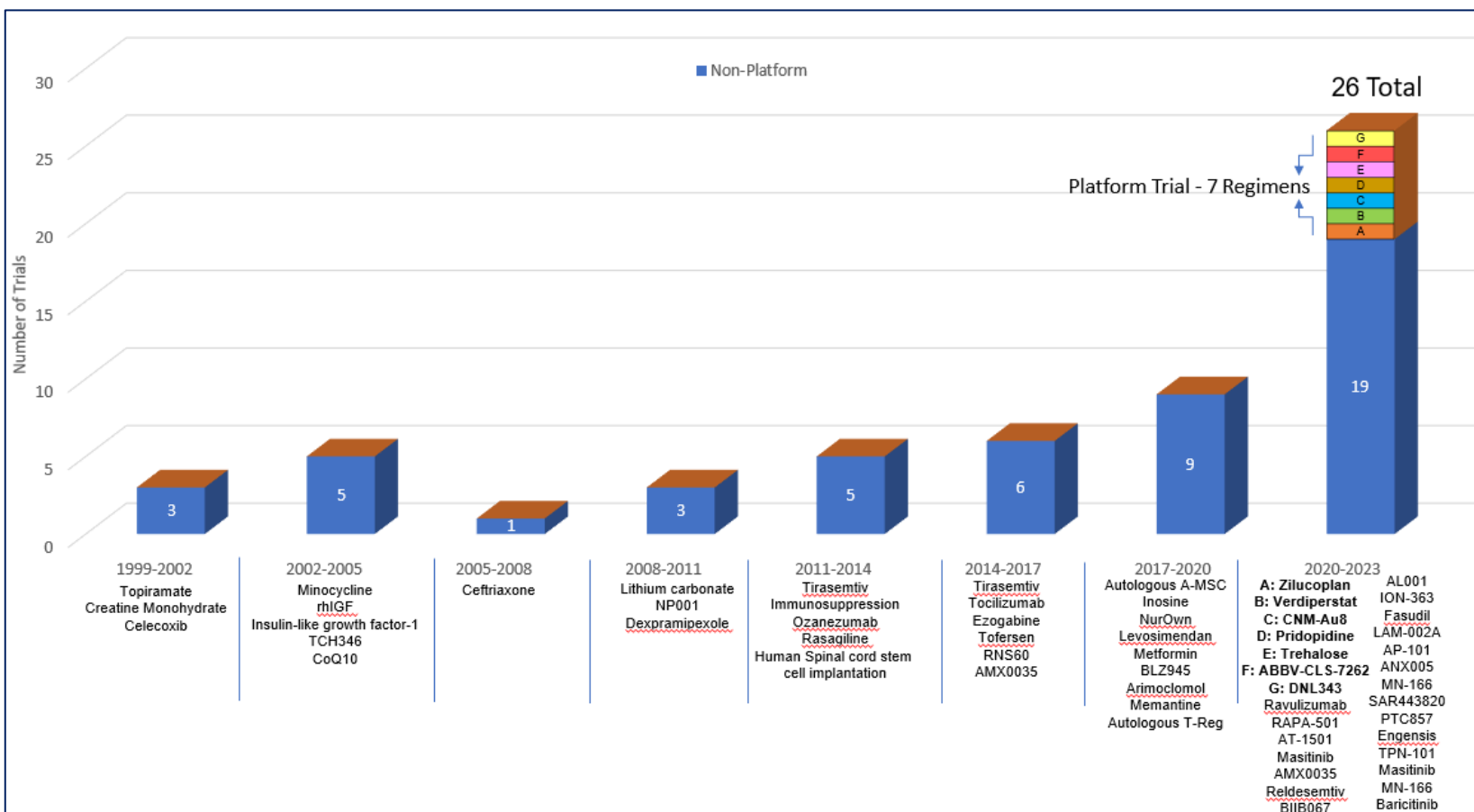
- **Gene carriers**

- **Reverse ALS**

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

The ALS drug development pipeline is robust

ALS Clinical Trials Enrolling in the US (Phase 2, 2/3, 3)



Note: Trials are listed within the date range in which enrollment began

The evolving landscape of ALS clinical trials

Current Opportunities and Challenges:

- Efficient trials
 - to prioritize and rapidly move drugs that are more likely to succeed to phase 3
- Biomarker discovery and validation
 - key to identify individuals who are more likely to respond to specific agents and make trials even more efficient
- Research capacity
- Access and inclusion
- Academic networks
- Global regulatory considerations
- Access to approved drugs
- Real-world evidence, clinical effectiveness studies

The HEALEY ALS Platform Trial

A Multistakeholder Partnership to Accelerate ALS Drug Development



Healey Center

Sean M. Healey & AMG Center
for ALS at Mass General



Calico



alsFINDINGaCURE™

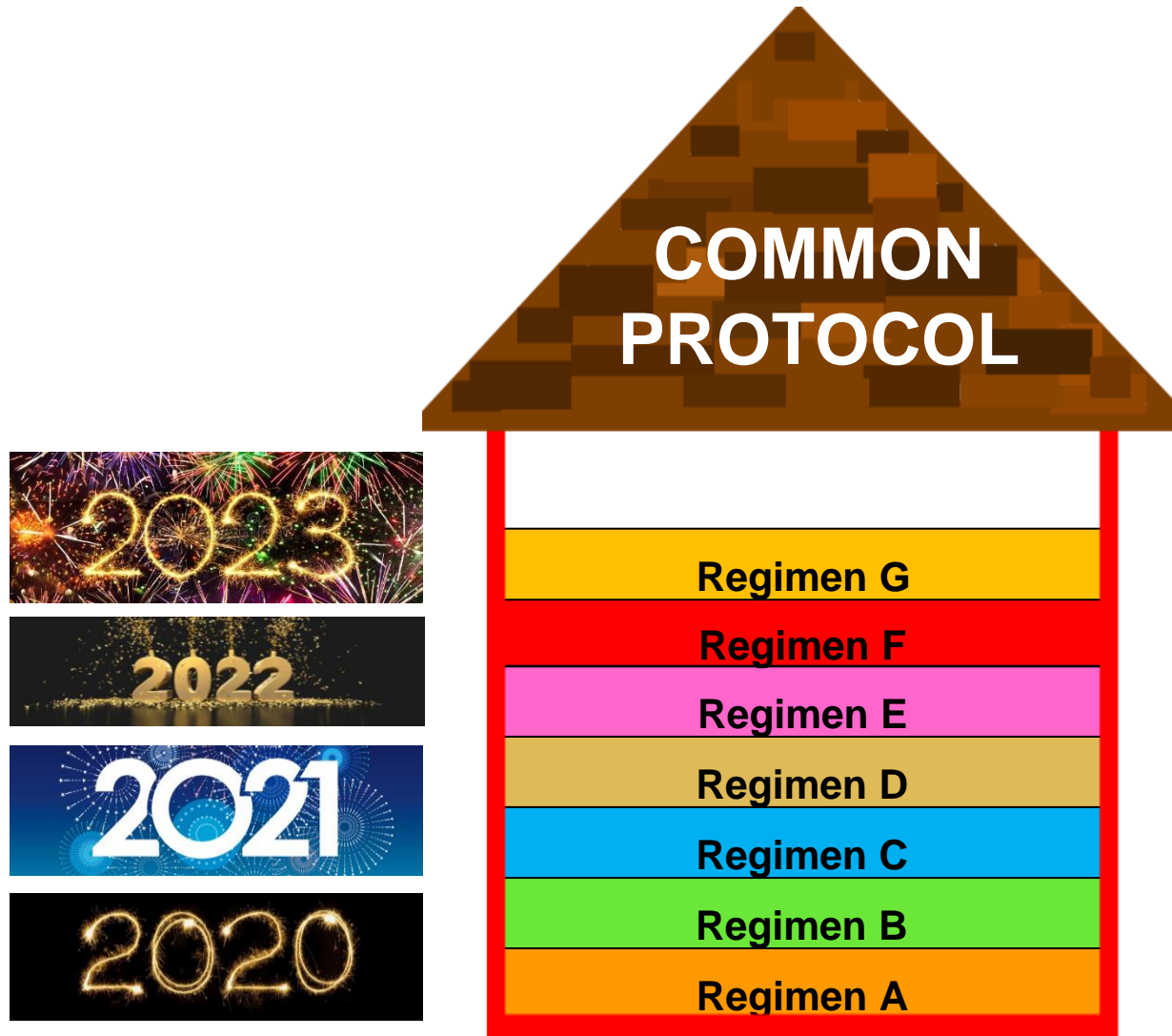


THE ARTHUR M. BLANK
FAMILY FOUNDATION

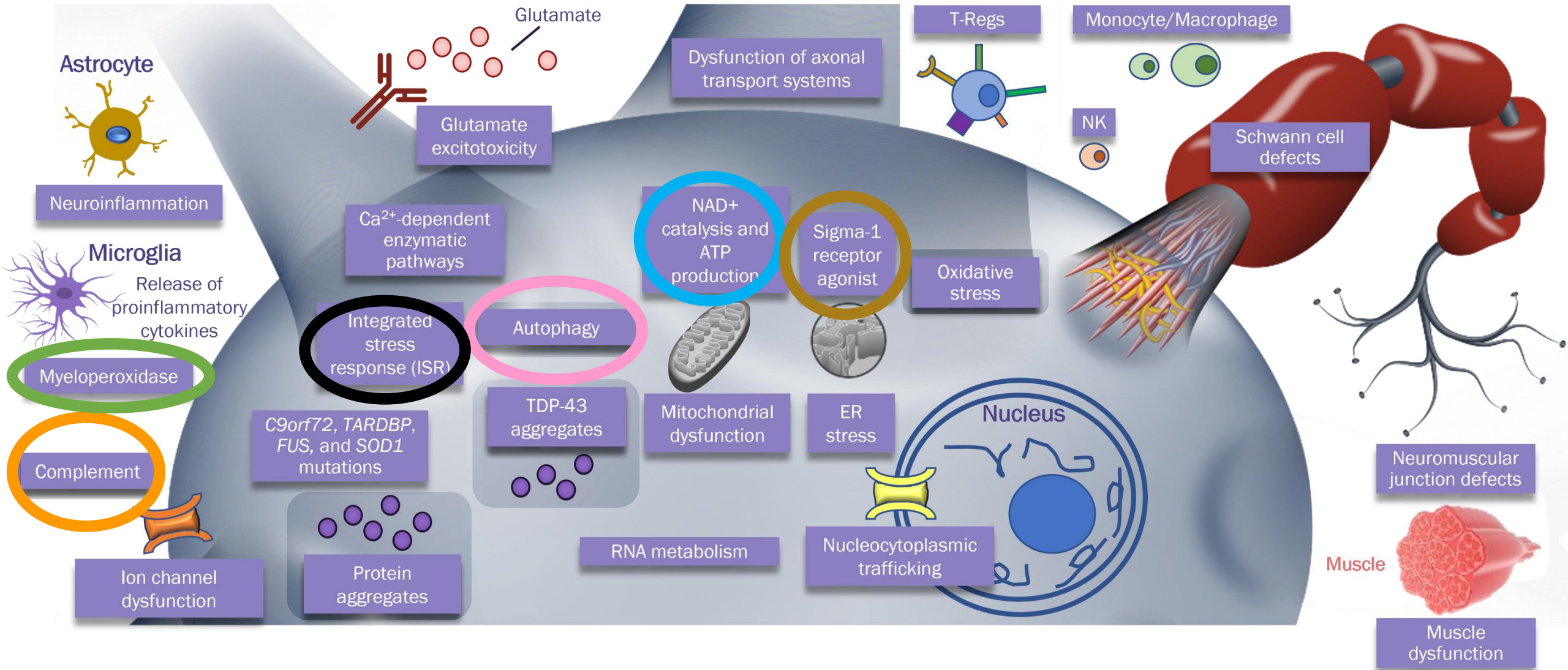


The AMG Foundation

Common Protocol and Shared Infrastructure Allow for Operational and Scientific Efficiencies



Regimen: Active Study Drug + Matching Placebo



Patient Navigation

Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



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

ALS Link sign-up:



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

Upcoming Webinars:

May 25th- Weekly Q&A and overview of Platform Trial progress to date

June 1st- Weekly Q&A



Allison Bulat