

# HEALEY ALS Platform Trial

Weekly Q&A – Jan 13, 2022



## Healey Center

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



The AMG Foundation

# Guest Speaker

**Sharon Hesterlee, PhD**  
Chief Research Officer  
Muscular Dystrophy Association (MDA)



# Working to Defeat ALS

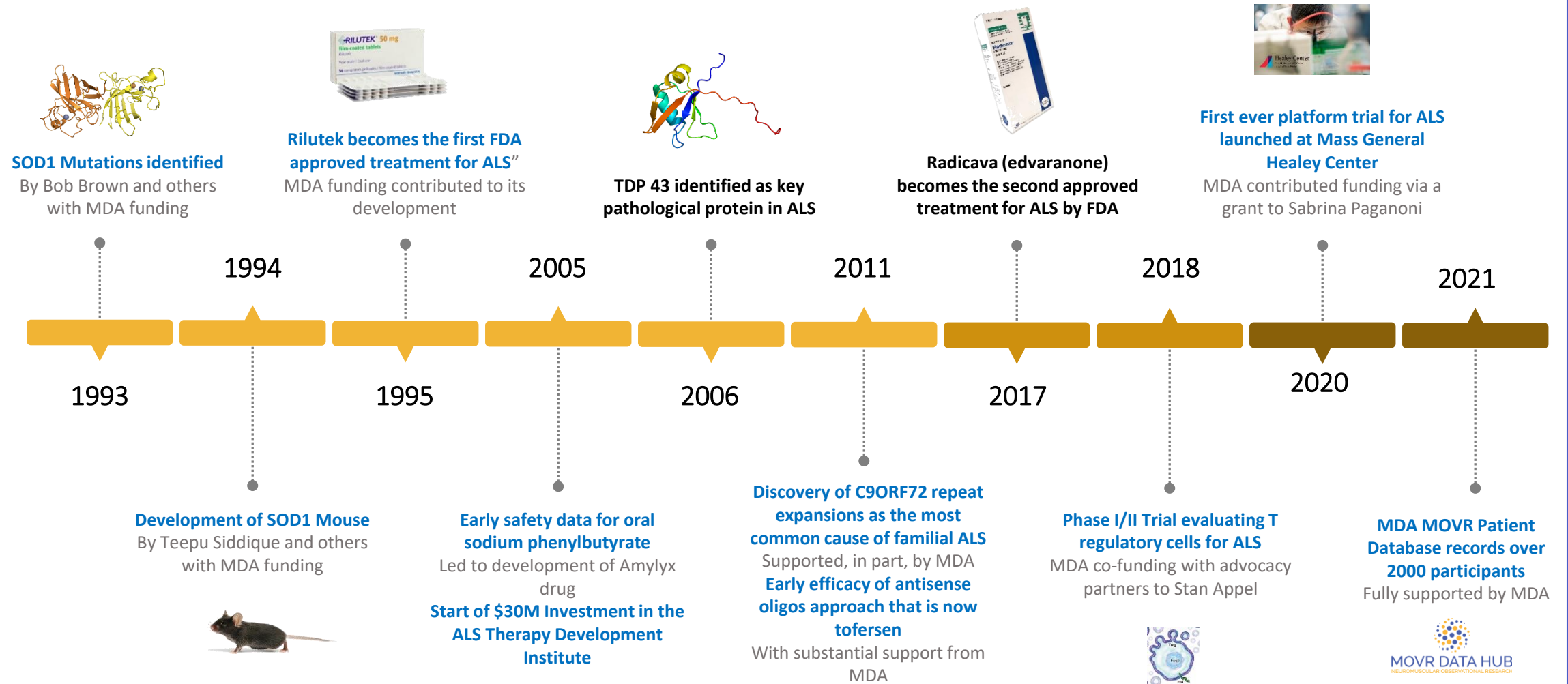
The Muscular Dystrophy Association is an Umbrella Organization Covering Over 300 Neuromuscular Diseases, including ALS

1/13/2022



# \$170M investment by MDA has fueled critical breakthroughs in ALS

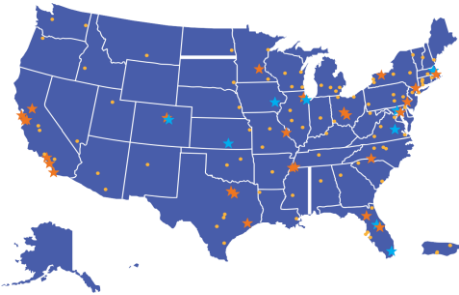
In the 1950's Eleanor Gehrig requested MDA's help in the fight against ALS



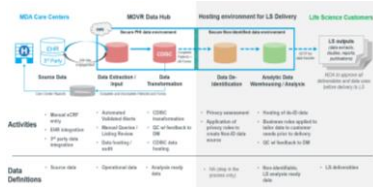


# MDA Integrated Research and Clinical Resources

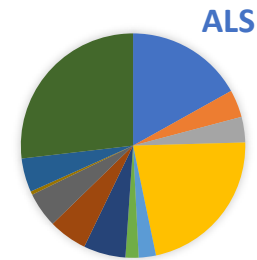
**MDA Care Center Network:**  
150 sites provide 90,000+ medical visits annually for more than 60,000 individuals



**MOVR Data Hub:**  
HIPAA, FAIR data principals and CDISC compliant; data collected at point-of-care for ALS, DMD, BMD, FSHD, LGMD, SMA, and Pompe



**Research Grants Program:**  
Over \$1B invested in neuromuscular research  
Over \$170M invested in ALS



**Industry Relationships:**  
Over 60 active collaborations around data, insights, clinical trials and education



- Professional Education:**
- CME
  - Best Practice Sharing
  - Treatment/Care Updates
  - MDA Monthly Report news
  - Advisory Meetings
  - COVID19 materials



**Annual Professional Conference – in 2021:**

- 900 Registered attendees
- 140 speakers
- 92 Abstracts Submitted
- 50 exhibitors
- 30 sessions with CME Credits
- 12 Industry Forums
- [Register free for virtual attendance](https://forms.office.com/r/H1zTnjvgSw)

**Scientists and Clinicians:**  
MDA has funded over 7000 unique investigators and trained 2000 young scientists



# MDA ALS Focus: Care, Research and Data

## Care



MDA Maintains 150 neuromuscular specialty Care Centers across the US-

48 are focused specifically on ALS

## Research

\$170M in ALS Research Grants Made to Date

Example of Research Grant: ALS Platform Trial: Accelerating the Path to Effective Treatments

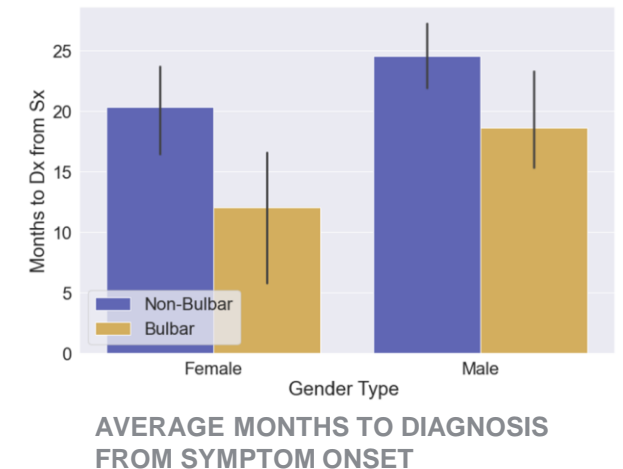
Sabrina Paganoni, MD, PhD



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## Data

MDA MOVR Data Hub contains rigorous clinical data from 2000 Participants with ALS



# MDA ALS Focus: Advocacy and Education

## Advocacy

[www.votervoice.net/MDA/home](http://www.votervoice.net/MDA/home)

**Three Leading ALS Organizations  
Celebrate President Biden Signing  
the ACT for ALS into Law  
December 23, 2021**



### Current Issues



#### Access to Care and Therapies from Day 1

Early detection and intervention of neuromuscular diseases is critical in order to deliver adequate and appropriate healthcare. [Continue reading →](#)



#### Accelerating Therapy Development

We are committed to working with key stakeholders, including the US Food and Drug Administration (FDA), to help accelerate development of life-changing therapies. [Continue reading →](#)



#### Empowerment and Independence

MDA works every day to ensure that members of the neuromuscular community are able to learn, work, travel, and socialize without discrimination. [Continue reading →](#)

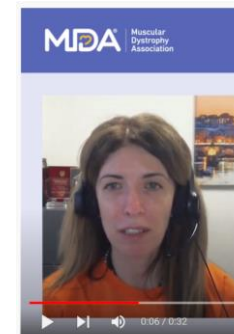


#### MDA Advocacy Statements and Communications to Policy Makers

Learn more about MDA's communication with policy makers. [Continue reading →](#)

## Education

[www.mda.org.community-ed](http://www.mda.org.community-ed)



MDA Facebook Live Event: Care and Research Update

ALS Care and Research Update Facebook Live Event

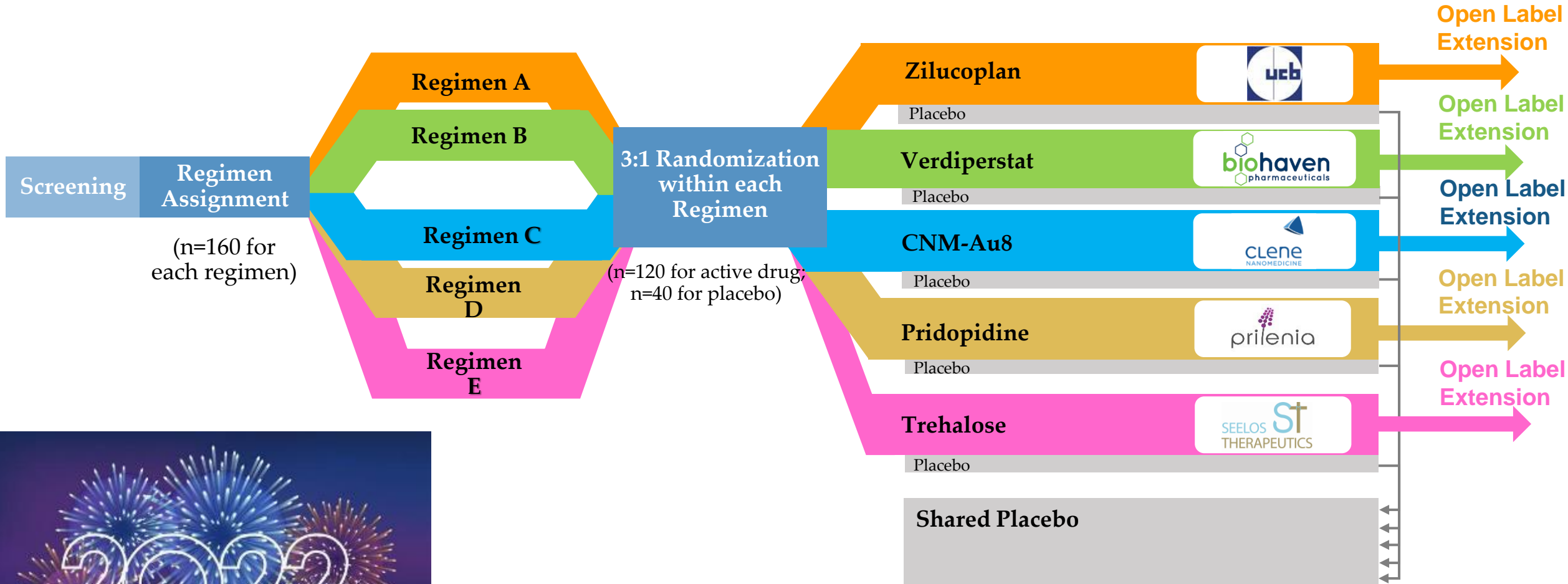
### **MDA Engage: Amyotrophic Lateral Sclerosis (ALS)**

May 12-13, 2021

Live, Virtual Webinar

[Watch Recorded Seminar Here](#)

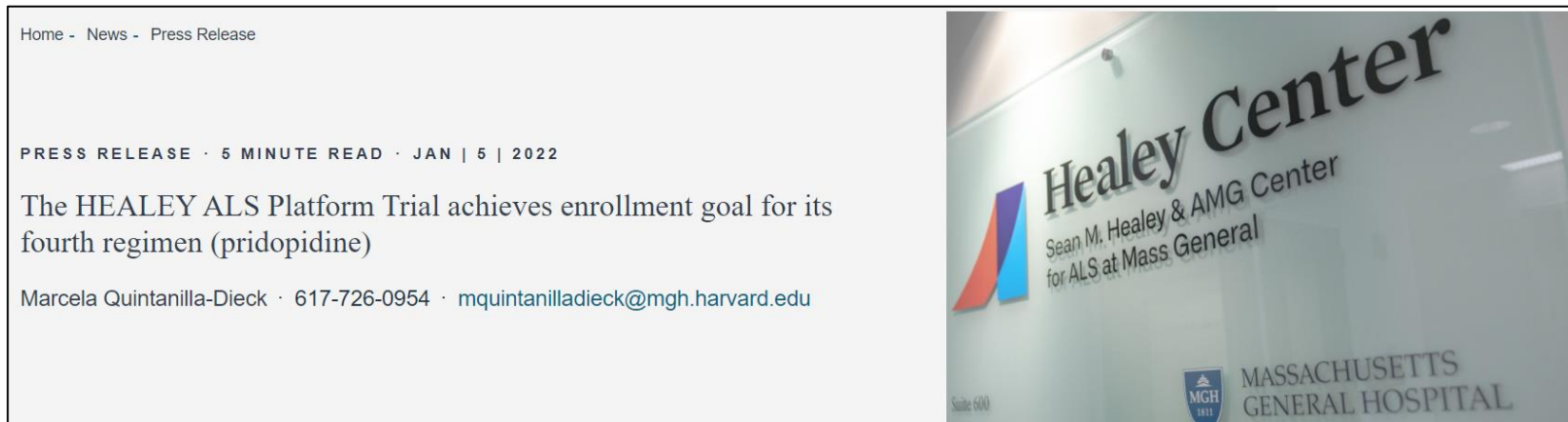
# The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial





# Regimens A, B, C and D completed enrollment!

- ✓ **162** individuals were randomized within Regimen A
- ✓ **167** individuals were randomized within Regimen B
- ✓ **161** individuals were randomized within Regimen C
- ✓ **163** individuals were randomized within Regimen D



**> 300 participants have entered the Open Label Extension (OLE)**

***= Thank You =***

**This breakthrough trial would not be possible without your partnership**

# Send us webinar ideas!

## Upcoming Guest Speaker:

Jan 20<sup>th</sup>- Senda Ajroud-Driss, MD (Northwestern University, Chicago IL)

Weekly webinar  
registration:



ALS Link sign-up:

