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
$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

- **1993**: SOD1 Mutations identified
  - By Bob Brown and others with MDA funding

- **1994**: Rilutek becomes the first FDA approved treatment for ALS
  - MDA funding contributed to its development

- **1995**: Development of SOD1 Mouse
  - By Teepu Siddique and others with MDA funding

- **2005**: TDP 43 identified as key pathological protein in ALS

- **2006**: Early safety data for oral sodium phenylbutyrate
  - Led to development of Amylyx drug
  - Start of $30M investment in the ALS Therapy Development Institute

- **2011**: Discovery of C9ORF72 repeat expansions as the most common cause of familial ALS
  - Supported, in part, by MDA
  - Early efficacy of antisense oligos approach that is now tofersen
  - With substantial support from MDA

- **2017**: Phase I/II Trial evaluating T regulatory cells for ALS
  - MDA co-funding with advocacy partners to Stan Appel

- **2020**: MDA MOVR Patient Database records over 2000 participants
  - Fully supported by MDA

- **2021**: First ever platform trial for ALS launched at Mass General Healey Center
  - MDA contributed funding via a grant to Sabrina Paganoni

MDA MOVR Patient Database records over 2000 participants
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

**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/H1zTnjygSw)

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

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

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

Data

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

Average Months to Diagnosis from Symptom Onset
MDA ALS Focus: Advocacy and Education

**Advocacy**

www.votervoice.net/MDA/home

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

**Education**

www.mda.org.community-ed

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

Regimen A
(n=160 for each regimen)

Regimen B

Regimen C

Regimen D

Regimen E

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Trehalose
Placebo

Shared Placebo

Open Label Extension

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Open Label Extension
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 20th- Senda Ajroud-Driss, MD (Northwestern University, Chicago IL)

Weekly webinar registration:  ![QR Code](image)

ALS Link sign-up:  ![QR Code](image)