

HEALEY ALS Platform Trial

Weekly Q&A – Sept 2, 2021



Healey Center

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



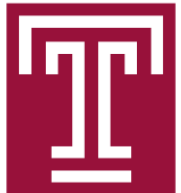
The AMG Foundation

Guest Speaker

Terry Heiman-Patterson, MD
Temple University, PA
Platform Trial Site Investigator



MDA ALS Center of Hope at Temple University Lewis Katz School of Medicine



ALS is a family affair!



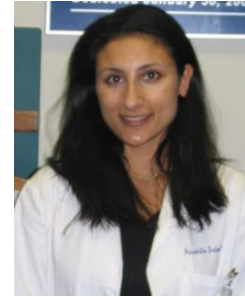


Treatment of ALS Takes a Team Approach

We are the Extended Family!

Multidisciplinary Approach

- PT/OT
- Assistive Technologist
- Speech and Language Pathology
- Nutrition
- Respiratory therapy
- Psychotherapy
- Nursing
- Research



Symptom Management

Disease modification

Encouraging Clinical Research



MDA/ ALS Center of Hope
Commitment to Care
Progress through Research and Collaboration

Multidisciplinary Clinic

Basic Research Lab

Clinical Activities

Clinical Research

Clinical Care and Educational Programs

Clinical Trials

Pre Clinical Drug Development

Genetic Modifiers

Tissue Bank

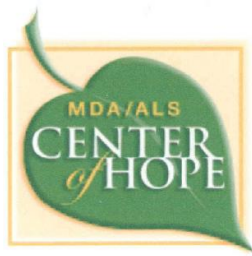
Respiratory measures and care
Natural History
Oxidative Stress
GI motility
Assistive Technology and BCI

Platform Trial
Amylyx-Phoenix
Oral Radicava
Masitinab
Refine
Cytokinetics

Testing of potential therapeutics in Animal models
Development of Biomarkers of disease phenotype

G93A SOD1
Dynactin

Target ALS Program
Autopsy Specimens
Serum, plasma, DNA, Urine



MDA/ALS Center of Hope Basic Research Program



Genetic Modifiers of ALS Mice

- We have identified genetic regions on Chr17 in SOD1 mice that modify the severity of illness in the SOD1 mouse and also in mice with other motor neuron diseases
- Translational Consortium: We are working with a group of investigators to develop a program to translate our findings regarding modifiers of disease in mice to humans
- These modifiers will serve as targets for treatment, help us understand ALS and motor neuron diseases, and may provide biomarkers of disease

Oxidative Stress in ALS and Effects of anti-oxidant medication

Biomarker Initiatives

Maintenance of a Tissue Bank and Autopsy bank



ALS Center of Hope: Clinical Research Program

Clinical Trials

- Healey Platform Trial
- Phoenix and Centaur
- MT-1186-A01 and A02
- Cytokinetics Reldesemtiv
- Masitinab
- Chromolyn
- Phenotype Differences in Response to Radicava

Clinical Research

- Biomarkers (Target ALS, Refine)
- Tissue Bank
- Natural History Studies (ALS, PLS, MOV'R)
- Philadelphia Respiratory Consortium
- Oxidative Stress in ALS
- GI motility
- Health Outcomes and Cost Analysis for PALS and Clinics
- **BCI/Assistive Technology**
 - Technology for independent control/communication
 - Use and Needs Technology Survey



Leveraging Technology While We Work on the Cure

Develop and Test the Next Generation of Technology

Home Based Systems for Brain Computer Interfaces (EEG based p300)

Expand Functionality

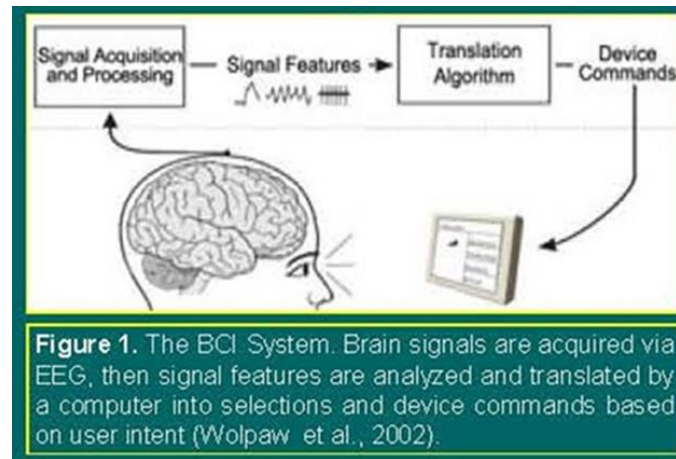
Environmental control

Long term goal of mobility

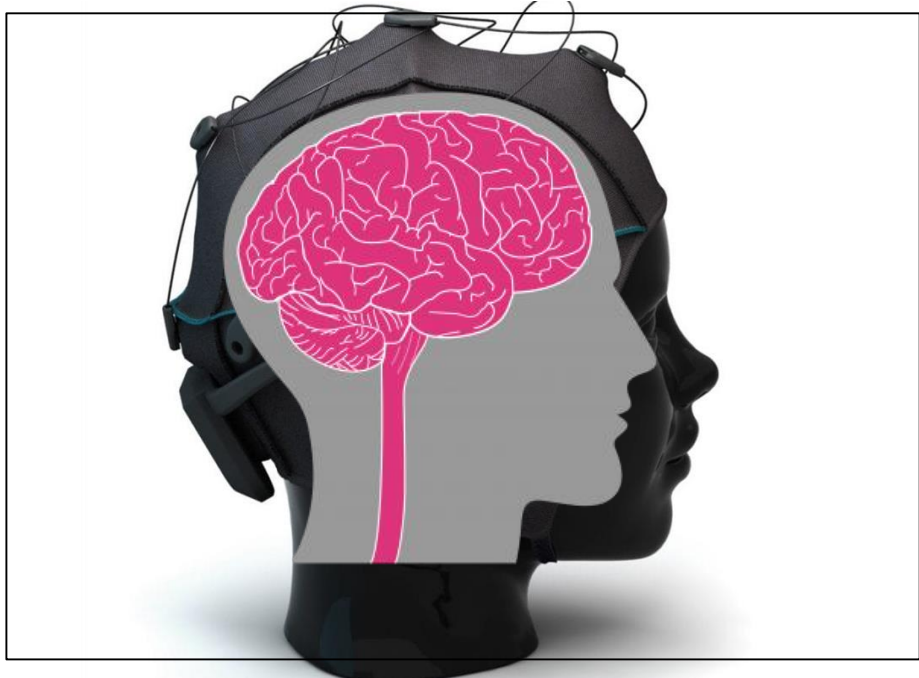
Create an independently useable simple system

Control on –off switch accessible by the user

Use affordable off the shelf commercially available components



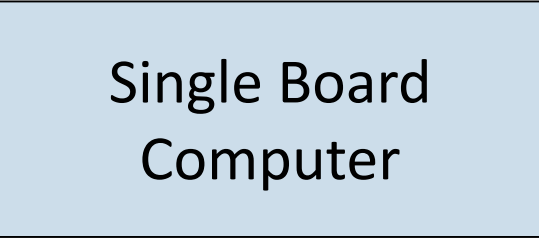
Solution Pathway: Simplification



EEG Headset

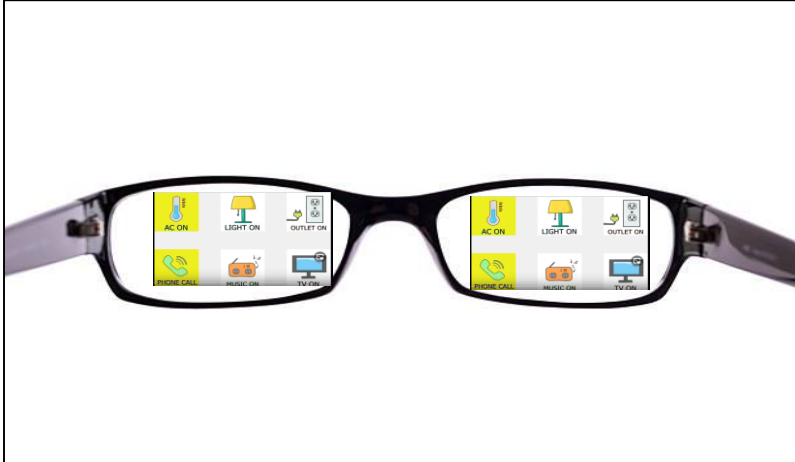


- Raspberry Pi 4-B
- 8 GB RAM
- 1.5 GHz processor
- USB connection
- Bluetooth
- ~\$60



Main-processing Computer

Modified AR



- TFT LCD
- 1.8 inch diagonal
- 0.352 ounces
- 128 x 160 pixels
- 30 FPS
- ~\$10

- Anker Battery Pack 26800
- 26800 mAh portable charger
- USB power output
- Display charge in 10% intervals
- ~\$45

Prototype has been built!



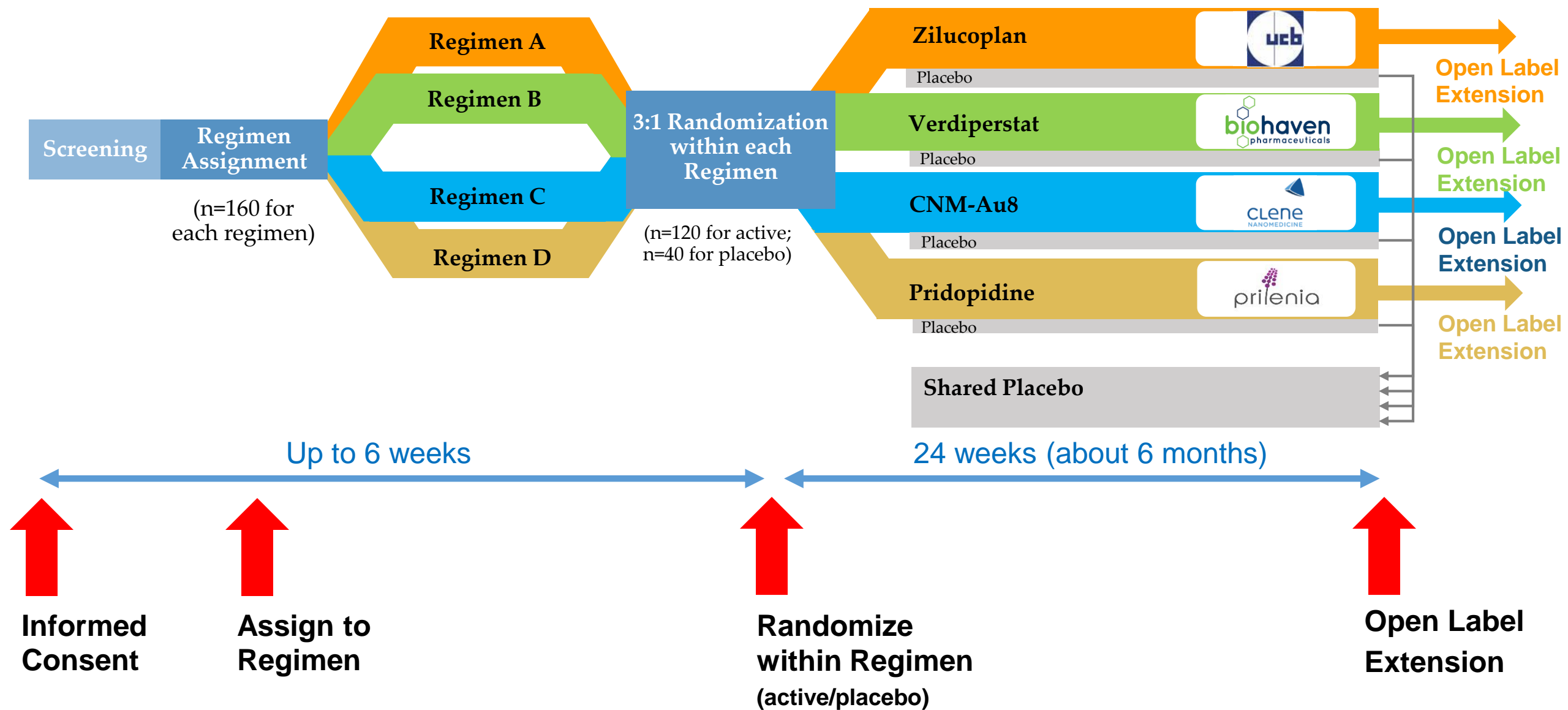
Accuracy in Test
Group: 81%

- Plans in process:
 - Develop Bluetooth connections
 - Develop independent control
 - Beta test on PALS

Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of September 2, 2021)

- **724** individuals with ALS signed informed consent
- **575** individuals were assigned to a regimen
- **523** individuals were randomized within a regimen (active or placebo)
- **195** have entered the Open Label Extension (OLE)

- | | Total randomized +
Participants in screening |
|---|---|
| • 142 individuals were randomized within Regimen A | = 152 |
| • 158 individuals were randomized within Regimen B | = 164 |
| • 158 individuals were randomized within Regimen C | = 161 |
| • 65 individuals were randomized within Regimen D | = 69 |

Thank You

This breakthrough trial would not be possible without your partnership

Patient Navigator



Catherine Small

Allison Bulat

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To see whether you might qualify,
view the list of eligibility criteria:

<https://bit.ly/3p4PrLL>



Send us webinar ideas!

- Biomarkers
- Biostatistics / Trial Design
- Get to know our sites



Upcoming Guest Speakers:

Sept 9th- Jennifer DiMartino (Executive Director, ALS ONE)

Sept 16th- No webinar this week in observance of Yom Kippur

Sept 23rd- Namita Goyal, MD (University of CA Irvine, CA)

Oct 7th- No webinar this week