Guest Speaker

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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
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 AO2
- 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

Figure 1. The BCI System. Brain signals are acquired via EEG, then signal features are analyzed and translated by a computer into selections and device commands based on user intent (Wolpaw et al., 2002).
Solution Pathway: Simplification

EEG Headset

Single Board Computer

Main-processing Computer

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

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

Modified AR

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

Screening

Regimen Assignment
(n=160 for each regimen)

Regimen A
Regimen B
Regimen C
Regimen D

3:1 Randomization within each Regimen
(n=120 for active; n=40 for placebo)

Regimen D

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

Up to 6 weeks
24 weeks (about 6 months)

Informed Consent
Assign to Regimen
Randomize within Regimen (active/placebo)
Open Label Extension
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

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