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.
HEALEY ALS Platform Trial

Weekly Q&A – July 13, 2023
ALS Clinic & Research Program at OSU

Sarah Heintzman, MS, APRN-CNP, FNP-C, CCRC
Neuromuscular Division

July 13th, 2023
About Me

- 11 years of experience working in neuromuscular neurology at OSU currently as a Nurse Practitioner

- 17 years of experience working in clinical research in the following roles:
  - Clinical research coordinator
  - Senior research coordinator
  - Research nurse
  - Research manager
  - Sub-investigator
  - Principal investigator

- Co-chair of the HEALEY ALS Platform Trial Recruitment and Retention Committee, a member of the Cure SMA Care Center Network Committee, and is the Clinic Coordinator for the MDA and ALS clinics at OSU

- Certified clinical research coordinator as recognized by the Association of Clinical Research Professionals

- Spare time:
  - SCUBA, swimming, snorkeling
  - Mom to my 9 year-old son, Sam
  - Gardening, reading, travel & playing with my dog, Petey
The Team at OSU

Stephen J. Kolb, MD, PhD; ALS/MND Program Director

Adam Quick, MD

Jennifer Roggenbuck, LGC

Cheyenne Grove, ALS Research Program Senior Coordinator

▪ Respiratory Therapy: Jerry Reynolds
▪ Social Work: Kinsey Ludt
▪ Occupational Therapy: Theresa Berner
▪ Physical Therapy: Andrea Jaworek
▪ Clinic Nurse: Alison Potter
▪ Registered Dietician: Kathryn Jira
▪ Speech Language Pathologist: Audrey Hall
▪ Research Coordination: Kaneshia Hives
Integrating Research into the Clinic

- Would you like to have a conversation about research?
- Knowledge is power
  - What is a clinical trial?
  - How do clinical trials help?
  - What is it like to be in a clinical trial?
  - How do I sign up for a clinical trial?
  - What if I decide I want to quit being on a clinical trial?
  - Who pays for a clinical trial?
- Discuss open studies at OSU
- Discuss the science - where are things headed?
Integrating Research into the Clinic

Smart Phrases in Epic

- Information can be easily updated
- Cost effective – no formal printing required
- Is printed out in the after visit summary

ALS Clinical Trials

The doctors, nurses, and researcher coordinators in Neurosurgical Neurology are committed to ensuring that patients, caregivers, and patients groups are involved in all aspects of disease research from the planning stage to the follow-up stage. The goal is to ensure that patients are not only receiving the best care possible, but also have the opportunity to participate in clinical trials. Clinical trials can help find new and better ways to prevent and treat ALS.

What is a clinical trial?

A clinical trial is a research study that follows a specific set of rules (protocol). The trial is designed to test disease treatments on an experimental basis. The treatments tested may involve new drugs, new doses of existing drugs, new surgical procedures, new medical devices, biological agents, psychological procedures, etc.

How do clinical trials help?

Testing a new treatment or therapy that may help you and others may help improve treatment for patients in the future. Being part of a clinical trial may give you access to new treatments that are not available to patients unless they are part of a clinical trial. Clinical trials are done in phases, with each phase testing the treatment in different groups of people. People who participate in a clinical trial may help us find new treatment methods sooner.

What is it like to be in a clinical trial?

- You will be given detailed information about the clinical trial to help you decide if you want to participate. A member of the healthcare team will explain the study being done and why it is important. You and your family will be asked to sign a consent form to participate in the study.
- If you decide to participate, you will be assigned to a clinical trial protocol. You will be asked to follow the protocol.
- If it is important for you to follow the clinical trial protocol, an informed consent form will be signed by you and/or your legal representative.
- The protocol will describe:
  - Exactly which clinical trial will be open
  - Which clinical trials are ongoing
  - What is being tested in each trial
  - How often you should be chosen to participate
  - What is the clinical trial coordinator or investigator

How do I sign up for a clinical trial?

Talk with a member of your healthcare team if you are interested in participating in a clinical trial. If you are interested in participating in a clinical trial that has been approved, you may be eligible to take part.

What if I decide I want to quit being on a clinical trial?

If you decide that you no longer want to participate in a clinical trial, you may stop at any time. If a person leaves a trial, they should talk to their doctor about other treatment options.

Who pays for a clinical trial?

Generally, there are no costs associated with participating in a clinical trial. You and your caregiver may also receive a stipend for your time. The stipend varies by trial and is based on the amount of time you spend participating in the trial.

ALS Association (ALS)

- 1-800-ALSA and ALS Signal - Clinical Research Dashboard - To move promising treatments forward until we find cures.
- https://www.mda.org/als/clinical-trials
- https://als.org/clinician-resource-center/als-research
- http://www.mda.org/als/clinical-trials
- https://als.org/als-association-alsi.org
- http://als.org/research/
Integrating Research into the Clinic

- Open and active listening
- Discuss goals and expectations for trials
  - From trial perspective
  - From patient perspective
- Be mindful of the ALS Clock
Preparing for a Clinical Trial:

- Medical Records including the following:
  - Clinic notes from last neurologist office visit
    - This often will include the most recent ALSFRS-R scores
  - Any genetic test results
  - Results of pulmonary function testing
Things to think about:

- Selecting a trial
  - How long will the trial last?
    - Generally around 26 weeks (6 months)
  - What is the medication and its mechanism of action?
    - Talk with your doctor
  - What route: oral, IV, subcutaneous, intrathecal

- Commitments to the study
  - Time (length and frequency of the visits)
  - Tests
The sunflower is a symbol of strength, grace and dignity. When in clusters, the flowers support each other, allowing for growth in a multitude of shapes, sizes and colors. This diversity represents the people fighting this disease and reminds us to always have hope.
HEALEY ALS Platform Trial:

- Regimen A
- Regimen B
- Regimen C
- Regimen D
- Regimen E
- Regimen F
- Regimen G

Common Protocol and Shared Infrastructure

Screen for eligibility ➔ Randomization 3:1 ➔ Active

Screening Period ➔ Randomized Period 24 weeks ➔ Open-Label Extension Period (Active Treatment Extension)

ENROLLMENT COMPLETE

ENROLLING

ENROLLING
Enrollment Update: Regimen F (as of 7/13/23)

Participants consented to Master Protocol since RGF initiated

Participants assigned to RGF

Participants randomized within RGF (enrollment goal ≈240)

Thank You
for your partnership in ALS research
Enrollment Update: Regimen G (as of 7/13/23)

129
Participants consented to Master Protocol since RGG initiated

26
Participants assigned to RGG

20
Participants randomized within RGG (enrollment goal ≈240)

Thank You
for your partnership in ALS research
Checking Site Status Online

List of Participating Sites

Sites marked "Recruiting" are currently enrolling participants. Sites marked "Active, Not recruiting" are active in the Platform Trial (for example, they are following participants in ongoing regimens that have already completed enrollment) but are not enrolling new participants at this time.

<table>
<thead>
<tr>
<th>Site</th>
<th>State</th>
<th>Enrollment Status</th>
<th>Trial Contact Information</th>
</tr>
</thead>
<tbody>
<tr>
<td>Barrow Neurological Institute</td>
<td>AZ</td>
<td>Recruiting</td>
<td>Whitney Dailey</td>
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<tr>
<td>Cedars-Sinai Medical Center</td>
<td>CA</td>
<td>Recruiting</td>
<td>Sophia Mostowy</td>
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<td>Forbes Norris MDA/ALS Research Center,</td>
<td>CA</td>
<td>Recruiting</td>
<td>Teji Dulai</td>
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<td>California Pacific Medical Center</td>
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<td>Kaiser Permanente, Los Angeles Medical</td>
<td>CA</td>
<td>Recruiting</td>
<td>Mary H. Berganza</td>
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<tr>
<td>Medical Center</td>
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</tbody>
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Regimen G Drug Science Q&A Webinar

Topic: DNL343 Drug Science and Mechanism of Action
Link to Register: https://bit.ly/3NqJU1j

Open to everyone!
Thursday, July 20th
5:00-6:00pm Eastern
Patient Navigation
Central resource for people living with ALS

Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

Upcoming Webinars:
July 20th - Regimen G / DNL343 Drug Science Q&A Webinar with Denali
August 3rd - with guest host Suma Babu, MBBS, MPH (Mass General Hospital)