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.
Enrollment Update: Regimen F and Regimen G

382
Participants consented to Master Protocol since RGF and RGG initiated

318
Participants assigned to RGF or RGG

272
Participants randomized within RGF or RGG

Thank You
for your partnership in ALS research

(as of 10/12/23)
63 Sites Currently Active

(as of 10/12/23)
### 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>
</tr>
<tr>
<td>Cedars-Sinai Medical Center</td>
<td>CA</td>
<td>Recruiting</td>
<td>Sophia Mostowy</td>
</tr>
<tr>
<td>Forbes Norris MDA/ALS Research Center, California Pacific Medical Center</td>
<td>CA</td>
<td>Recruiting</td>
<td>Teji Dulai</td>
</tr>
<tr>
<td>Kaiser Permanente, Los Angeles Medical Center</td>
<td>CA</td>
<td>Recruiting</td>
<td>Mary H. Berganza</td>
</tr>
</tbody>
</table>

[Contact a study team near you to discuss enrollment opportunities](https://bit.ly/3g2NZr5)
Assessing emerging biomarkers/outcome measures

**DNA** – genetic analysis for all regimens

**Neurofilaments** – NfL analysis for all regimens

**Biomarkers** (Blood, Urine, CSF) – several drug-specific biomarkers in each regimen

**Speech Analysis** – data collected for regimens A-D

**Home Spirometry** – critical during the pandemic for regimens A-D

Additional biomarkers/outcome measures being considered for upcoming regimens (PBMCs, ROADS)
HEALEY ALS Platform Trial
Innovation through Biomarkers
Learn how your participation makes a difference
JULY 2023

What is a Biomarker?
A biomarker is an indicator of what is happening inside of your body. Biomarkers include everything from vital signs to laboratory tests of blood and other tissues.
A biomarker may be used to: (1) learn more about a disease, (2) follow disease progression, (3) monitor how a disease responds to a study drug, or (4) serve as a target for treatment.

How do your biomarkers contribute to ALS research?
In the HEALEY ALS Platform Trial, biomarkers are studied in your blood, urine, and cerebrospinal fluid (CSF), or assessed via digital technology.
Neurofilaments (NF) are an example of a biomarker found in blood and CSF that has shown great potential in monitoring disease progression and drug effectiveness. NF levels are higher in the blood and CSF of people with ALS. This can be attributed to the breaking down of motor neurons, which spill contents such as neurofilaments into the spinal fluid. By analyzing your biofluids, the research team is able to gather information on changes in important biomarkers, including NF levels. These analyses enable the team to have a better-informed view of how the study drug works, which may influence the course of drug development and result in a long-lasting impact on ALS research.

Blood Samples
Blood carries nutrients, drugs, and wastes throughout the body. Blood samples allow researchers to measure how the study drug is broken down and used in your body. In the HEALEY ALS Platform Trial, monitoring changes in various protein, hormone, and NF levels over time may help determine the effectiveness of an investigational drug.

Urine Samples
Urine samples enable researchers to collect valuable biomarkers as they are flushed out of your system. For example, p75 is a protein which increases in concentration with ALS progression. Tracking this protein may help determine drug efficacy and disease progression in certain trials.

Cerebrospinal Fluid (CSF) Samples
CSF, the fluid that surrounds the brain and spinal cord, is in direct contact with motor neurons and the central nervous system. Given its location, we can measure breakthrough biomarkers that are not available to be measured in the blood. CSF samples are collected through lumbar punctures (LP). Scan the QR codes below to learn more about lumbar punctures.

At-Home Digital Biomarkers
Recent innovation in digital technology has allowed for the collection of biomarkers from the comfort of your home. In the HEALEY ALS Platform Trial, some regimens may use various digital biomarkers. Examples may include:

Speech Analysis
Voice recordings are an example of digital biomarkers that may be collected in the HEALEY ALS Platform Trial. Speech recordings enable researchers to monitor variables such as pace and clarity of speech, and track whether any changes occur as a result of disease progression. Recordings of your voice may be collected during in-person study visits and at home via a smartphone app.

Spirometry
Spirometry is a lung function test that measures the quality and strength of your breathing. This test may be taken from home.

Stay Connected to ALS Research
Join the MGH ALS Link to Stay Connected to Research:

Contact Patient Navigator: HEALEYALSPlatform@mgh.harvard.edu or 833-425-8257 (HALT ALS)

For more information:

Register for HEALEY ALS Platform Trial Q&A Webinars:

https://www.mgh.harvard.edu/center-for-neurological-research/clinical-research/MEALS
This public webpage provides:

1. General Brochure and Biomarkers Brochure
2. Overview of science behind Regimen F and Regimen G
3. Links to download relevant brochures, including the Lumbar Puncture Brochure
4. Links to view relevant videos and webinar recordings

Regimen F: ABBV-CLS-7262, by Calico and AbbVie - Now Recruiting

- Watch a webinar about the science behind ABBV-CLS-7262
- Watch this video for more information on the mechanism of action behind ABBV-CLS-7262.
- Download Regimen F Brochure
- Download Lumbar Puncture Brochure
- Step by Step Guide to Lumbar Punctures: Watch short video

Regimen G: DNL343, by Denali Therapeutics

- Download Regimen G Brochure
- Watch a webinar about the science behind DNL343

https://www.massgeneral.org/neurology/als/research/first-platform-trial-treatments
Patient Navigation
Central resource for people living with ALS

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

Weekly webinar registration:
https://bit.ly/3r6Nd2L

ALS Link sign-up:
https://bit.ly/3o2Ds3m

Upcoming Webinars:
October 19th- weekly Q&A hosted by Merit Cudkowicz, MD MSc
October 26th- weekly Q&A
November 2nd- weekly Q&A and Regimen G science (tentative date)