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.
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.

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*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.

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Stay Connected to ALS Research

Join the MGH ALS Link to Stay Connected to Research:

https://lp.constantcontactpages.com/su/saTzwlp/ALSLink

For more information:

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

Register for HEALEY ALS Platform Trial Q&A Webinars:

https://www.massgeneral.org/neurology/als/research/platform-trial

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[QR Code for Lumbar Puncture Video](https://www.youtube.com/watch?v=3om1OEnlI8o)

[QR Code for Lumbar Puncture Brochure](https://www.massgeneral.org/assets/mgh/pdf/neurology/als/platformtrial_lumbarpuncture.pdf)