ANSWER ALS

1000 PARTICIPANTS REACHED

The enrollment goal of 1000 participants was met and exceeded by December 2018. Enrollment is closed but follow-up visits will continue. Thanks to all the patient participants who helped meet these goals and move research forward more quickly.

DATA PORTAL LAUNCHED

The first set of Answer ALS study data is now available to request at data.answerals.org! Just two months after reaching our target enrollment of 1000 participants, this release includes whole genome sequence data paired with full clinical profiles from the first 100 study participants. As profiles are completed, they will be added until the entire trove of data is available to the global ALS research community. Datasets can be searched based on both clinical and sample features allowing all researchers to rapidly identify the most relevant information for their own studies.

ANNUAL SCIENTIFIC MEETING HELD

- Preliminary results from multi-omics analysis of motor neuron lines derived from the first 100 participants demonstrated the potential for researchers to discern ALS sub-types. While encouraging, the omics team cautioned that these results need to be validated in the larger Answer ALS participant pool.

- Study participants with ALS can now choose to enroll in a sub-study that allows return of genetic sequencing results. Led by the Answer ALS clinical team at Ohio State University, the sub-study will give participants the option to learn if they carry one of the known ALS genes and/or genes that may increase the risk for certain cancers or heart conditions. Learn more at https://redcap.osumc.edu/redcap/surveys/?s=9DRH8MTHAT.

- Stem cell lines have been initiated for almost half of our study participants. As they clear quality control, these lines are openly available to the global research community through the core facility at Cedars-Sinai.

> "I have attended many many ALS meetings and this is the first one where I really felt true hope, I can see a little light shining at the end of the tunnel."
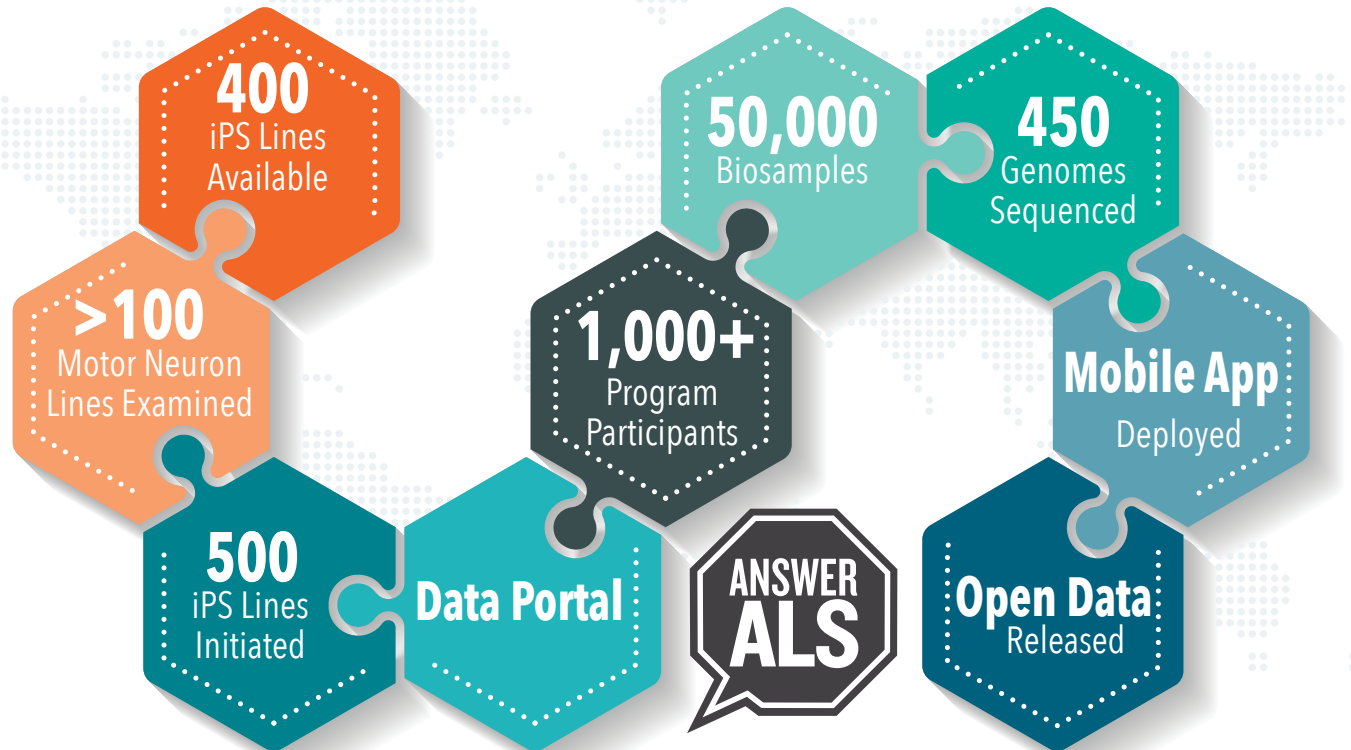>
> *ALS Researcher*

MORE UNPRECEDENTED MILESTONES

400 iPS Lines Available

50,000 Biosamples

450 Genomes Sequenced

>100 Motor Neuron Lines Examined

1,000+ Program Participants

Mobile App Deployed
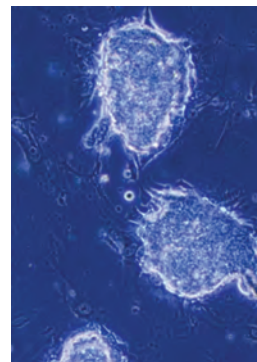
500 iPS Lines Initiated

Data Portal

ANSWER ALS

Open Data Released

Using Voice to Monitor Disease Progression

In partnership with IBM Research, Answer ALS scientists are using voice analytics to monitor disease progression. Preliminary results indicate that the voice recordings can be used to evaluate and monitor not only speech, but also cognition. Data gathered from additional PALS will be used to confirm these early results and we will provide additional details in the coming months.

Induced Pluripotent Stem (IPS) Cells: Did you know?

IPS cells need to be "fed" every day like their human donors! To stay happy and healthy, they receive a mixture of nutrients and growth factors that is refreshed every day of the year.

IPS cells were first generated in 2006 by a research team at Kyoto University in Japan.

Motor neurons were first generated from an 82- year-old woman with ALS in 2008.

GAME CHANGER GALA 2020

Stay tuned for more information about the 2020 Game Changer Gala in New Orleans. The tentative date is March 21st and the planners are working to create another extraordinary event honoring ALS Game Changers. www.GameChangerGala.com