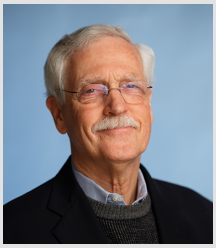
Year in Review of Studies Funded by ALS Finding a Cure 2019–2020

Successful Readout for Amylyx's CENTAUR ALS Clinical Trial

The start-up company Amylyx announced that its CENTAUR ALS clinical trial, a 6-month double-blind, placebo-controlled study in 137 ALS participants, had successfully met its primary endpoint to delay progression in sporadic ALS patients. The trial was led by Drs. Sabrina Paganoni and Merit Cudkowicz, ALS Finding a Cure® investigator and Chief Medical Officer respectively. The ALS Association collaborated in supporting Amylyx as part of the ALS ACT initiative. The first trial manuscript is currently under review at a major journal. Amylyx will discuss the results of this study and next steps with the FDA in the first half of 2020.

Major Progress in Advancing Gene Therapy for ALS



Dr. Robert Brown, Director of the Program in Neurotherapeutics at the University of Massachusetts Medical School, and lead investigator for the AAV10-SOD1 initiative has successfully administered a SOD-1 targeted gene therapy developed by his team to a second SOD1-ALS patient under a Compassionate Use provision. Dr. Brown also co-founded a company, Apic Bio, to advance the therapeutic potential of the gene therapy approach, initially enabled by ALS Finding a Cure® funding, and the company has raised enough funding to launch a multi-center trial.

Answer ALS Study Completes Targeted Enrollment of over 1000 ALS Patients and Controls

The Answer ALS project successfully achieved its target enrollment, enrolling more than 1000 ALS patients and controls in the world's largest ALS cross-platform molecular analysis initiative to date. The majority of participants have had genetic sequencing of their DNA performed and induced pluripotent stem cell lines created in order to enable molecular analysis, with the goal of identifying distinct disease subgroups. Data from the first 100 iPS cell lines/patients, representing the largest collection of complete biological ALS data, was released late April 2020. Data and samples from the study are being made available to the ALS research and pharma communities to accelerate disease discoveries.

HEALEY ALS Platform Trial

ALS Finding A Cure®, in partnership with Tackle ALS, is providing support for the first platform trial in ALS, an approach to accelerate therapy development and greatly increase access to treatments for people with ALS.

The Dominantly Inherited ALS (DIALS) Initiative Awarded Funding to Expand Enrollment

The DIALS initiative was launched to identify the earliest signs of disease in presymptomatic carriers of known ALS genes. The study is open to enrollment and 87 participants have been enrolled to date. In recognition of the importance and utility of this project, Target ALS and The ALS Association, as well as some private donors, have awarded the DIALS project an additional $56K to enable increased enrollment of ALS gene carriers. Industry support for the project is now being considered, highlighting the value of the DIALS initiative for both understanding and developing treatments for the genetic forms of ALS.

Phase II Study of Expanded Autologous T-regulatory Cells

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The goal of this project is to test the ability of an ALS patient's own T-regulatory immune cells to be extracted from blood, expanded in the laboratory, and injected back, in hopes this will slow disease progression (as assessed by ALSFRS-R). The Phase IIa protocol was approved by the FDA, and the double-blind, placebo-controlled trial was initiated at Massachusetts General Hospital and Houston Methodist Hospital. To date the trial has enrolled 7 participants. These studies, co-funded by the ALS Association, and the Muscular Dystrophy Association, have led to several new pharma companies launching T-regulatory programs for ALS.

Assessing the Microbiome in People with ALS

The Microbiome Project was designed to test the hypothesis that changes in the intestinal microbiome could be relevant in ALS. A methodology was developed to sequence the gut microbiome in people with ALS. Investigators detected surprising differences between the composition of bacterial species present in the gut of people with ALS versus those present in healthy and disease controls. The investigators have now submitted a manuscript of their findings and launched two new collaborations to build upon their initial results, potentially culminating in a clinical trial.

Success by the numbers

8
Clinical trials initiated by ALSFAC funded investigators

120
Academic partners engaged

320
ALS patients treated

4000
Samples collected from patients