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RESEARCH

EFFICIENCY: ALS patients cannot afford to wait.

The partnerships forged by ALS ONE add efficiency and improved coordination to the fields of ALS research and care, increasing the speed of discovery and reducing the duplication of efforts. The ALS ONE patient network creates a direct pipeline between therapy discovery and immediate implementation and patient services.

- The Neurological Clinical Research Institute (NCRI) at MGH serves as the Coordination Center for ALS ONE clinical trials, as well as the Coordination and Data Management Center for the Northeast ALS Consortium, a group of 100+ academic centers worldwide dedicated to finding new ALS therapies.
- With the goal to accelerate trial initiation, NCRI established the first (and only) central IRB for ALS trials. ALS trials may now begin an average of 7-12 months earlier than before. The effort to grow the reach of this central IRB nationally is already underway.
- NCRI is working to create big data for ALS by collecting, merging, and housing the largest ALS clinical trials dataset ever created. NCRI also manages a world-renowned repository of blood and spinal fluid samples from various research studies of ALS and other motor neuron diseases, with over 70,000 samples available to researchers.
- CCALS and the MGH ALS clinic have streamlined a common release form, allowing enhanced communication between care and treatment teams and increasing referrals for high-quality care.
- ALS ONE-affiliated teams across institutions regularly communicate and share data, discuss findings, and establish best practices. They are consistently committed to linking lab-based findings to patient care and treatment.

INNOVATION: Researching cutting edge ALS treatments.

ALS ONE-affiliated researchers are performing innovative research and collaborating together to quickly translate new findings from the lab to patients.

- **Innovations in Gene Therapy:** Since discovering the first ALS-caustic gene over 20 years ago, Dr. Robert Brown of UMass Medical Center has worked to develop gene therapies to treat people with ALS. Through an ALS ONE partnership with MGH, the team is now altering disease genes in patients using a harmless virus delivered in a single dose, potentially revolutionizing care and treatment for people with genetic forms of ALS.
- **Targeting Inflammation: A Novel Approach:** Researchers conducting ground-breaking science from ALS TDI and creating innovative trial designs and new outcome measures from NCRI are partnering to target inflammation in ALS. In people with ALS, regulatory immune cells are depleted, and inflammation in the brain and spinal cord causes disease progression and paralysis. ALS TDI researchers are developing a drug to restore the immune system balance and tamp down inflammation; thus far it has shown significant benefit in an animal model of ALS. Meanwhile, a team at MGH has developed new imaging tools to measure inflammation in patients with ALS. The ALS ONE partnership has hastened the development of this promising anti-inflammatory therapy from the lab to patients, and preclinical work is underway.
- **Novel Measurements to Quickly Detect Treatment Efficacy:** Traditional measures of clinical efficacy require time-consuming and expensive ALS clinical trials. The MGH ALS imaging group is developing novel tools to measure the biological effect of experimental treatments in a way that can reduce the trial size from 400 to 30 patients and the trial duration from 12 to 3 months. This added efficiency allows ALS ONE to test many more therapies quickly.



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CARE

ACCESS

Every ALS patient deserves equal access to high-quality care and treatment options

Over half of ALS patients never see an ALS specialist. Our new Access ALS Initiative will link people with ALS to the best quality care and treatment options, not just in Massachusetts, but worldwide. Our partnerships with medical centers across the country enable us to provide specialized services remotely, and our innovative programs to increase access can be replicated to ensure that every patient is able to benefit from specialized care and treatment, regardless of location or income level.

- CCALS referrals from MGH, ALS TDI, and UMASS Medical Center have increased by 38 percent from 2016 to 2017 as a result of the ALS ONE partnership between the organizations. Not only are patients better able to access quality in-home care as a result, but they are provided with streamlined information about relevant clinical trials at these institutions and transportation assistance to attend clinical trials.
- CCALS and the MGH ALS clinic have launched the ALS House Call program to take care and research opportunities beyond the walls of the clinic to the level of the home, where people need it most, and insurance often doesn't cover the costs of providing care.
- The MGH ALS program is staffed with a Research Access Nurse, who participates actively in educating patients about research opportunities at ALS ONE-affiliated institutions, engaging with the patient community regarding new treatments, and pre-screening patients for trials. The nurse has thus far taken over 500 calls from those seeking information. This model has been well-received by the patient community and has increased efficiency for trial recruitment; it may be a model to expand upon nationwide with appropriate funding.

