

Accelerating Progress in ALS

Support from The McCourt Foundation

October 2022

At the Sean M. Healey & AMG Center for ALS at Mass General, we take a collaborative approach to understanding amyotrophic lateral sclerosis (ALS) at the molecular level and work toward translating those discoveries to promising therapies. Support from The McCourt Foundation has provided critically important seed funding for innovative research in our laboratories focused on basic science as well as clinical research that moves those laboratory discoveries into patients, helping us move more quickly to the cures.

HEALEY ALS Platform Trial

The HEALEY ALS Platform trial is a revolutionary new approach to evaluating therapies that have shown promise in the laboratory and are ready to be tested in people. This trial allows us to test several drugs at the same time, reduces the time it takes to evaluate a drug's effectiveness by 50 percent and reduces the cost of a trial by 30 percent. The most exciting aspect of the HEALEY ALS Platform Trial is that unlike traditional approaches, this trial allows us to add or drop therapies once their effectiveness is determined without the need to stop and restart the trial.

Launched in July 2020, enrollment has been faster and larger than any other ALS trial with more than 1,000 individuals consented to participate, 813 assigned to a regimen and 479 enrolled in Open Label Extensions. More than 70 centers across the United States are part of the HEALEY ALS Platform Trial network, ensuring close and broad access to people with ALS. Results from the first four compounds tested in our game-changing HEALEY ALS Platform Trial are being reviewed now – just two years after we launched the trial in the midst of COVID restrictions. We already added a fifth compound and with philanthropic support, hope to add more compounds each year. The enthusiasm from patient and industry partners will help us increase the number of therapies being studied at a given time as well as more rapidly identify the most effective treatments. With this tool, testing can continue until cures are found.

Research Access Nurse and Patient Navigator

The Mass General ALS Research Access Nurse (RAN) and Patient Navigator (PN) have connected with thousands of people with ALS (PALS) in need of direction throughout the world, guiding them to research opportunities and expediting enrollment in trials. As the complexity and volume of clinical research increases, the RAN and PN roles are increasingly important to help PALS find highly personalized research opportunities. The RAN and PN play a critical role in the Healey Center's outreach activities and tracking this activity.

The success of the RAN and PN is due in large part to the personalized attention they provide. They ensure that every caller receives a timely response, thoughtful answers and clear direction. They also

develop educational webinars, website materials including posting webinar recordings, and other patient-facing resources. In short, their role has been critical to the growth we are experiencing. As the RAN and PN are often the PALS' first point of contact with the Healey Center, they are strategically positioned to expand our reach.

In 2021 alone, the RAN and PN fielded calls and emails from nearly 1,600 individuals, while the webinars were attended by 3,907 individuals and the recordings were viewed by an additional 9,144. The opportunity to stream or host recorded webinars allows us to share information with PALS without regard to geographic boundaries.

Our goal is to expand this team to include a second Research Access Nurse, while also providing an additional support staff member to ensure we can appropriately triage calls and emails as they come in and continue to provide timely responses and appropriate guidance.

Impact of Your Support

Philanthropic support from our community partners, like The McCourt Foundation, allows Healey Center researchers the resources to collaborate across geographic and scientific boundaries to pursue groundbreaking research that will make a meaningful difference to individuals with ALS and their families. The dedicated support from fundraisers like you, provides a unique opportunity to contribute to the next wave of scientific breakthroughs and plays a pivotal role in our quest to cure ALS.

Together we will find the cures.