

October 2023

## Healey & AMG Center for ALS Spearheads Expanding Access to Investigational Drugs

At the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital, we are dedicated to developing effective treatments for ALS and to providing people with ALS access to experimental therapies through our Expanded Access Protocol (EAP) programs. Since our launch in 2019, we partner with several stakeholders to create new EAP models for ALS. Our goal is to create access to promising, new therapies for those individuals with ALS who are not eligible for clinical trials. The research data that is generated from these EAPs can also be useful in understanding ALS and potentially aid in bringing new therapies to market.



401  
*Total Participants*



26  
*Unique Sites*



17 *Investigational Products*

### Expanded Access Partnerships



[TechVsALS](#) is a fundraising campaign founded by Shawn Sarbacker, a 30-year tech industry veteran and person with ALS. In collaboration with the Healey & AMG Center, TechVsALS works to encourage companies, foundations, organizations, and individuals to join them in sponsoring EAP programs nationwide.

Thanks to TechVsALS in partnership with Kulicke & Soffa and Massachusetts General Hospital a new EAP Program was established at Thomas Jefferson Hospital Weinberg ALS Center to provide patients in the Philadelphia area with the access to ALS investigational treatment. Read more about the program [here](#)

## The axeALS Foundation in partnership with the Sean M. Healey & AMG Center for ALS establishes Expanded Access Protocol (EAP) Program at the University of California, Irvine

The axeALS Foundation is partnering with the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital to establish a new EAP program at the University of California, Irvine, under the direction of, Namita Goyal, MD, professor of neurology, UCI School of Medicine, and director of UCI Health ALS Services.

[Read the full article](#)

## The Healey EAP Companion program is growing to more sites nationwide!



*The EAP program has opened doors for patients, research, and innovation in ALS care and we look forward to furthering this success at clinics across the US.*

## Latest News from the EAP program

### Sean M. Healey & AMG Center for ALS awarded NIH U01 Grant to support Expanded Access to Pridopidine in Collaboration with Prilenia Therapeutics

The Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital has been awarded a grant from the National Institutes of Health (NIH) - National Institute of Neurological Disorders and Stroke (NINDS) to conduct an intermediate size Expanded Access Protocol (EAP) in Amyotrophic Lateral Sclerosis (ALS) in collaboration with Prilenia Therapeutics.



[Read the full article](#)

### **Sean M. Healey & AMG Center for ALS awarded NIH U01 Grant to support Rapa Therapeutics' Expanded Access Protocol of Epigenetically Reprogrammed RAPA-501**

The Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital has been awarded a three-year grant to support Rapa Therapeutics' intermediate size Expanded Access Protocol (EAP) in Amyotrophic Lateral Sclerosis (ALS) from the National Institute of Neurological Disorders and Stroke (NINDS) at the National Institutes of Health (NIH). This EAP will evaluate the benefits of Rapa Therapeutics' (Rapa) investigational product RAPA-501, an Epigenetically Reprogrammed Autologous Hybrid T<sub>REG</sub>/Th2 T-Stem Cell Therapy, in people living with ALS (PALS).

[Read the full article](#)

### **Sean M. Healey & AMG Center for ALS and the University of California, Irvine Announce First Participant Enrolled in Expanded Access Protocol Program for Trehalose**

The University of California, Irvine (UCI) successfully enrolled the first participant in its newly established Expanded Access Protocol (EAP) program. Under the direction of Namita Goyal, MD, Professor of Neurology, UCI School of Medicine, and Director of UCI Health ALS Services, the EAP program allows people with ALS who do not qualify for trials to access investigational drugs that are not yet approved by the FDA. UCI's EAP program is made possible by support from the [axeALS Foundation](#) in partnership with the [Sean M. Healey & AMG Center for ALS](#) at Massachusetts General Hospital.

[Read the full article](#)

### **The Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital in conjunction with Seelos Therapeutics and NINDS announced the first patient-dosing in the NINDS-funded Expanded Access Protocol for Trehalose**

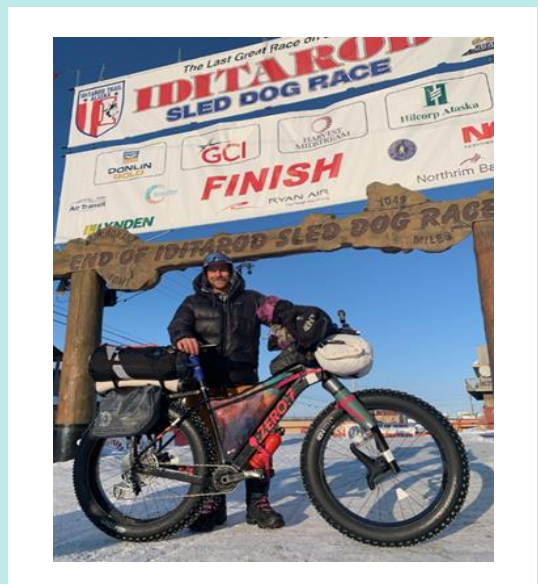
The trehalose ALS EAP allows for individuals with ALS who are otherwise not be eligible for ongoing clinical trials to access this investigational drug. Additionally, this EAP will provide real world safety, biomarker, and clinical data in individuals with ALS treated with long duration of trehalose.

[Read the full article](#)

## **In the Community**

On February 26<sup>th</sup>, Miron Golfman set out on the 1,000 mile Iditarod Trial in Noam Alaska as part of the Iditarod Trial Invitational. Miron participated this year to raise awareness and funds in support of the Expanded Access Protocol Program at the Healey & AMG Center in honor of his uncle, Bruce Rosenblum. He biked the entire trail, and completed the ride on Wednesday, March 15<sup>th</sup>. An incredible accomplishment!

To learn more, or support Miron's fundraising efforts, visit [ridetoendure.com](http://ridetoendure.com)



## Publications

- [Expanded access protocol \(EAP\) program for access to investigational products for amyotrophic lateral sclerosis \(ALS\)](#)  
Yerton M, Winter A, Gelevski D, Addy G, Kostov A, Lieberman C, Weber H, Doyle M, Kane G, Cohen C, Parikh N, Burke KM, Rohrer M, Stirrat T, Bruno M, Hochman A, Luppino S, Scalia J, D'Agostino D, Sinani E, Yu H, Drake K, Hagar J, Sherman AV, Babu S, Berry JD, Cudkowicz ME, Paganoni S. Muscle Nerve. 2023 Mar 16. doi: 10.1002/mus.27819. Online ahead of print.PMID: 36929648
- [Safety and activity of anti-CD14 antibody IC14 \(atibuclimab\) in ALS: Experience with expanded access protocol](#)  
Gelevski D, Addy G, Rohrer M, Cohen C, Roderick A, Winter A, Carey J, Scalia J, Yerton M, Weber H, Doyle M, Parikh N, Kane G, Ellrodt A, Burke K, D'Agostino D, Sinani E, Yu H, Sherman A, Agosti J, Redlich G, Charmley P, Crowe D, Appleby M, Ziegelaar B, Hanus K, Li Z, Babu S, Nicholson K, Luppino S, Berry J, Baecher-Allan C, Paganoni S, Cudkowicz M. Muscle Nerve. 2022 December 19. doi:10.1002/mus.27775
- [An expanded access protocol of RToo1 in amyotrophic lateral sclerosis- Initial experience with a lipid peroxidation inhibitor](#)  
Yerton M, Winter A, Kostov A, Lieberman C, Gelevski D, Weber H, Doyle M, Kane G, Parikh N, Burke K, Rohrer M, Stirrat T, Bruno M, Hochman A, Luppino S, Scalia J, Skoniecki D, D'Agostino D, Sinai E, Yu H, Sherman A, Babu S, Berry J, Midei M, Milner P, Cudkowicz M, Paganoni S. Muscle Nerve. 2022 October. vol. 66,4 (2022): 421-425. doi:10.1002/mus.27672

