



Current ALS Clinical Trials in the US

An abstract background graphic consisting of a complex network of interconnected nodes and lines, resembling a molecular structure or a data network. The nodes are small black dots, and the lines are thin, light gray. The network is dense and spans across the right side of the slide, with some nodes appearing more prominent than others.

<u>Treatment (Familial)</u>	<u>Recruiting</u>	<u>Enroll</u>	<u>Phase</u>	<u>Duration</u>	<u>Route</u>	<u>Open Label</u>	<u>Location(s)</u>
<u>Efficacy and Safety of Masitinib Versus Placebo in the Treatment of ALS Patients</u>						<u>AB Science</u>	
9/12/2023	Recruiting	495	Phase 3	24 Months (Anticipated)	Oral	No	Baltimore, MD, US Birmingham, AL, US View all 58
<u>A Study to Evaluate the Safety, Pharmacokinetics and Biodistribution of an Imaging Agent, 18F-OP-801 (18F Hydroxyl Dendrimer) in Patients With Amyotrophic Lateral Sclerosis (ALS) and Healthy Volunteers (HV)</u>						<u>Ashvattha Therapeutics, Inc.</u>	
9/10/2023	Recruiting	26	Phase1/2	0 Months (Anticipated)	Intravenous		Stanford, CA, US
<u>Studies in Amyotrophic Lateral Sclerosis (ALS) and Other Neurodegenerative Motor Neuron Disorders</u>						<u>Mayo Clinic</u>	
9/10/2023	Recruiting	3000		372 Months (Anticipated)	Blood Collection		Jacksonville, FL, US
<u>AMDX-2011P Retinal Tracer in Subjects With Neurodegenerative Diseases Associated With Amyloidogenic Retinopathy</u>						<u>Amydis Inc.</u>	
9/10/2023	Recruiting	36	Phase1/2	12 Months (Anticipated)	Intravenous		Newport Beach, CA, US Pasadena, CA, US
<u>A Single-Site Tissue Repository Providing Annotated Biospecimens for Approved Investigator-directed Biomedical Research Initiatives</u>						<u>Sanguine Biosciences</u>	
9/10/2023	Recruiting	20000		24 Months (Anticipated)	N/A		Waltham, MA, US
<u>Speech Accessibility Project</u>						<u>University of Illinois at Urbana-Champaign</u>	
9/10/2023	Recruiting	2000		12 Months (Anticipated)	N/A	N/A	Denver, CO, US Urbana, IL, US
<u>Feasibility of the BrainGate2 Neural Interface System in Persons With Tetraplegia</u>						<u>Leigh R. Hochberg, MD, PhD.</u>	
9/7/2023	Recruiting	2	N/A	36 Months (Anticipated)	Device	Unknown	Boston, MA, US Stanford, CA, US

Regulatory T Cells for Amyotrophic Lateral Sclerosis					Cellenkos, Inc.		
9/7/2023	Recruiting	66	Phase 1	48 Months (Anticipated)	Intravenous	Unknown	New York, NY, US
Mitochondrial Capacity Boost in ALS (MICABO-ALS) Trial					Dallas VA Medical Center		
8/26/2023 Combination Antioxidants	Recruiting	60	Phase 2	12 Months (Anticipated)	Oral	Unknown	Dallas, TX, US
A Study to Evaluate the Efficacy, Safety, Pharmacokinetics and Pharmacodynamics of ION363 in Amyotrophic Lateral Sclerosis Participants With Fused in Sarcoma Mutations (FUS-ALS)					Ionis Pharmaceuticals, Inc.		
8/26/2023	Recruiting	77	Phase 3	48 Months (Anticipated)	intrathecal	yes	Baltimore, MD, US Boston, MA, US View all 14
A Study of BIIB067 (Tofersen) Initiated in Clinically Presymptomatic Adults With a Confirmed Superoxide Dismutase 1 Mutation					Biogen		
8/26/2023	Recruiting	150	Phase 3	72 Months (Anticipated)	Not Available	yes	Atlanta, GA, US Austin, TX, US View all 29
A Study to Evaluate, Safety, Tolerability, Pharmacodynamic (PD) Markers and Pharmacokinetics (PK) of AP-101 in Participants With Amyotrophic Lateral Sclerosis (ALS)					AL-S Pharma		
8/26/2023	Recruiting	63	Phase 2	36 Months (Anticipated)	Intravenous	No	La Jolla, CA, US Leuven, Belgium View all 13
Neurodegenerative Alzheimer's Disease and Amyotrophic Lateral Sclerosis (NADALS) Basket Trial					Massachusetts General Hospital		
8/26/2023	Recruiting	20	Phase 1/2	24 Months (Anticipated)	Oral	Unknown	Boston, MA, US Charlestown, MA, US

CNS10-NPC-GDNF Delivered to the Motor Cortex for ALS**Cedars-Sinai Medical Center**

8/26/2023	Recruiting	16	Phase 1	12 Months (Anticipated)	Cellular transplant	No	Los Angeles, CA, US
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Study to Assess the Effects of PTC857 Treatment in Participants With Amyotrophic Lateral Sclerosis ALS**PTC Therapeutics**

8/26/2023	Recruiting	258	Phase 2	36 Months (Anticipated)	Oral	Yes	Augusta, GA, US Austin, TX, US View all 22
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Adenosine 2A Receptor Antagonism and AIH in ALS**University of Florida**

8/26/2023	Recruiting	40	Phase 1/ 2	24 Months (Anticipated)	Oral	No	Gainesville, FL, US
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Safety and Therapeutic Potential of the FDA-approved Drug Metformin for C9orf72 ALS/FTD**University of Florida**

8/26/2023	Recruiting	18	Phase 2	48 Months (Anticipated)	Oral tablet	Unknown	Gainesville, FL, US
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Study of Safety and of the Mechanism of BLZ945 in ALS Patients**Novartis Pharmaceuticals**

7/16/2023	Recruiting	56	Phase 2	84 Months (Anticipated)	Oral	Yes	Boston, MA, US La Jolla, CA, US View all 6
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A Study to Assess the Safety, Tolerability, and Effect on Disease Progression of BIIB105 in Participants With Amyotrophic Lateral Sclerosis (ALS) and Participants With the ALS Ataxin-2 (ATXN2) Genetic Mutation**Biogen**

6/16/2023	Recruiting	98	Phase 1/ 2	72 Months (Anticipated)	Unknown	No	Atlanta, GA, US Aurora, CO, US View all 17
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Evaluation of MN-166 (Ibudilast) for 12 Months Followed by an Open-label Extension for 6 Months in Patients With ALS**MediciNova**

6/12/2023	Recruiting	230	Phase 2/ 3	48 Months (Anticipated)	Oral	Yes	Allentown, PA, US Augusta, GA, US View all 24
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HEALEY ALS Platform Trial - Master Protocol					Merit E. Cudkowicz, MD			
5/17/2023	Recruiting	1500	Phase 2/ 3	72 Months (Anticipated)	Various	Yes	Allentown, PA, US Ann Arbor, MI, US View all 68	
Efficacy and Safety Extension Study of Oral Edaravone Administered in Subjects With ALS					Mitsubishi Tanabe Pharma America Inc.			
5/15/2023	Recruiting	300	Phase 3	24 Months (Anticipated)	Oral	No	Atlanta, GA, US Baltimore, MD, US View all 37	
RAPA-501 Therapy for ALS					Rapa Therapeutics LLC			
5/3/2023 RAPA-501 Autologous T cells	Recruiting	41	Phase 2/ 3	60 Months (Anticipated)	Oral	Unknown	Boston, MA, US Hackensack, NJ, US	
NuroSleeve Powered Brace & Stimulation System to Restore Arm Function					Thomas Jefferson University			
4/13/2023	Recruiting	20	N/A	60 Months (Anticipated)	Device	Not Available	Philadelphia, PA, US Wilmington, DE, US	
Remote Pulmonary Function Testing and Nurse Coaching in ALS					Milton S. Hershey Medical Center			
3/23/2023	Recruiting	40	N/A	48 Months (Anticipated)	Unknown	Unknown	Hershey, PA, US	
Investigation on the Cortical Communication (CortiCom) System					Johns Hopkins University			
2/23/2023 Device: Surgical implantation of CortiCom system	Recruiting	3	N/A	48 Months (Anticipated)	Surgical implantation of CortiCom system	Unknown	Baltimore, MD, US	

Also Still Recruiting

1/10/2023	BrainGate2: Feasibility Study of an Intracortical Neural Interface System for Persons With Tetraplegia	Leigh R. Hochberg, MD, PhD.					
Device	Recruiting	15	N/A	348 Months (Anticipated)	Device	Unknown	Atlanta, GA, US Boston, MA, US View all 5
12/6/2022	Non-invasive BCI-controlled Assistive Devices	University of Texas at Austin					
	Recruiting	40	N/A	24 Months (Anticipated)	Device	Yes	Austin, TX, US
10/17/2022	TJ-68 Clinical Trial in Patients With Amyotrophic Lateral Sclerosis (ALS) and Muscle Cramps	Hiroshi Mitsumoto					
	Recruiting	26	Phase 1/ 2	12 Months (Anticipated)	Powder in liquid	Unknown	Jacksonville, FL, US, New York, NY, US
9/13/2022	Optimizing BCI-FIT: Brain Computer Interface - Functional Implementation Toolkit	Oregon Health and Science University					
	Recruiting	60	N/A	36 Months (Anticipated)	Unknown	Unknown	Portland, OR, US
8/2/2022	ECoG BMI for Motor and Speech Control	University of California, San Francisco					
Device: PMT/Blackrock Combination Device	Recruiting	3	Phase Early 1	144 Months (Anticipated)	Device	Unknown	San Francisco, CA, US
7/8/2022	Remotely Supervised tDCS for Slowing ALS Disease Progression	University of Illinois at Chicago					
	Recruiting	100	N/A	24 Months (Anticipated)	Noninvasive brain stimulation	No	Chicago, IL, US
4/12/2022	Exploratory Evaluation of [11C]MPC6827	Columbia University					
	Recruiting	40	Phase Early 1	48 Months (Anticipated)	Oral	Unknown	New York, NY, US
3/16/2022	A Study to Evaluate the Safety and Pharmacokinetics of Single and Multiple Doses of Prosetin in Healthy Volunteers	ProJenX					
	Recruiting	32	Phase 1	0 Months (Anticipated)	Oral	No	San Antonio, TX, US
11/30/2020	Safety and Efficacy of Ranolazine for the Treatment of Amyotrophic Lateral Sclerosis	University of Kansas Medical Center					
Drug: Ranolazine	Recruiting	20	Phase 2	12 Months (Anticipated)	Oral tablet	Yes	Kansas City, KS, US
11/25/2020	Trial of Oxaloacetate in ALS	University of Kansas Medical Center					
	Recruiting	24	Phase 1	12 Months (Anticipated)	Oral	Unknown	Kansas, KS, US