

Exhibitors

Aeglea Bio Therapeutics (#908)

At Aeglea, we believe that every patient deserves a chance at a better life. We are committed to helping people with rare and devastating metabolic diseases who have limited treatment options because having a rare disease doesn't mean that you are in this fight alone.

Allergan, an AbbVie company (#914)

AbbVie's mission is to discover and deliver innovative medicines that solve serious health issues today and address the medical challenges of tomorrow. We strive to have a remarkable impact on people's lives across several key therapeutic areas: immunology, oncology, neuroscience, eye care, virology, women's health and gastroenterology, in addition to products and services across its Allergan Aesthetics portfolio.

Association of Child Neurology Nurses (ACNN) (#602)

The Association of Child Neurology Nurses is an international nonprofit organization of nurses and other health care professionals caring for children with neurological conditions. The ACNN provides educational opportunities at national and regional conferences, nursing excellence awards, research support, newsletters, and online membership contacts for networking. Additional information and how to join can be found at www.neurologynurses.org.

Astellas Gene Therapies Virtual

Astellas Gene Therapies is an Astellas Center of Excellence developing genetic medicines with the potential to deliver transformative value for patients. Based on an innovative scientific approach and industry leading internal manufacturing capability and expertise, we are currently exploring three gene therapy modalities: gene replacement, exon skipping gene therapy, and vectorized RNA knockdown.

BRONZE SPONSOR

AstraZeneca (#325)

AstraZeneca is a global, science-led biopharmaceutical company that focuses on the discovery, development and commercialization of prescription medicines in Oncology, Rare Diseases, and BioPharmaceuticals. Based in Cambridge, UK, AstraZeneca operates in over 100 countries, and its innovative medicines are used by millions of patients worldwide. Please visit astrazeneca-us.com and follow the Company on Twitter @AstraZenecaUS.



Atlantic Medical Group (#823)

Atlantic Health System, headquartered in Morristown, N.J., is an integrated health care system with a workforce of 18,000 members dedicated to building healthier communities. It includes 350 care sites and six hospitals. Our flagship hospital, Morristown Medical Center, is the #1 Hospital in NJ as ranked by US News & World Report, and is also home to Goryeb Children's Hospital.

SILVER SPONSOR

Biogen (#409) Boston & Virtual

At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies.



BioMarin Pharmaceutical, Inc. (#312)

BioMarin is a world leader in developing and commercializing innovative therapies for rare diseases driven by genetic causes. With a 20-year history, BioMarin remains steadfast to its original mission – to bring new treatments to market that will make a big impact on small patient populations. Visit www.biomarin.com to learn more.

BridgeBio (#723)

BridgeBio Pharma and affiliate Origin Biosciences, are dedicated to commercializing Nulibry® (fosdenopterin) for injection, an approved treatment for molybdenum cofactor deficiency (MoCD) Type A. We strive to deliver treatment to patients as quickly as possible because time is of the essence when facing the damage caused by this devastating disorder.

Children's National Hospital (#413)

Children's National Hospital, based in Washington, D.C., celebrates 150 years of pediatric care, research and commitment to community. Children's National is among the nation's top 10 children's hospitals ranking No. 1 for newborn care for the fifth straight year. In 2021, the Children's National Research & Innovation Campus opened, the first in the nation dedicated to pediatric research.

Cortica Virtual

Cortica is a pediatric healthcare organization focused on children with a wide range of neurodevelopmental differences. We aim to advance the standard of care, improve the family experience, achieve excellent patient outcomes, and unveil additional insights about this under-served population. All our Care Centers are led by pediatric neurologists and incorporate child neurology with occupational therapy, speech therapy, behavioral therapy, physical therapy, music therapy and family therapy.

Epilepsy Foundation New England (#608)

Epilepsy Foundation New England provides help to people and families affected by epilepsy in MA, NH, ME, and RI. Services include hospital, community based, and virtual Epilepsy Resource Rooms, care management, support groups, camps, financial assistance, advocacy, educational programs, & more. Come learn about the free supports available to patients.

GeneDx Inc. (#811)

GeneDx, Inc. is a global leader in genomics that provides genetic testing to patients and their families. Led by its world-renowned whole exome sequencing program, GeneDx has an acknowledged expertise in rare and ultra-rare genetic disorders, as well as one of the broadest menus of sequencing services available among commercial laboratories.

GOLD SPONSOR

Eisai Inc. (#408) Boston & Virtual

As the U.S. pharmaceutical subsidiary of Tokyo-based Eisai Co., Ltd., we are a fully integrated pharmaceutical business with discovery, clinical, and marketing capabilities. Our key areas of focus include oncology and neurology (dementia-related diseases and neurodegenerative diseases). To learn more about Eisai Inc., please visit us at www.eisai.com/US and follow us on Twitter and LinkedIn.



BRONZE SPONSOR

Genentech (#415)

For more than 40 years, we've been following the science, seeking solutions to unmet medical needs. As a proud member of the Roche Group, we make medicines to treat patients with serious medical conditions. We are headquartered in South San Francisco, California.



Elsevier, Inc. (#310)

Elsevier is a world-leading provider of information solutions that enhance the performance of science, health, and technology professionals, empowering them to make better decisions, and deliver better care.

Grace Science Foundation (#813)

Grace Science Foundation (GSF) is a research and patient advocacy organization. GSF sponsors 15 centers worldwide to understand and treat NGLY1 Deficiency, a rare multisystemic disorder. A gene therapy trial is scheduled for late 2022. The Foundation also works to identify patients

GOLD SPONSOR

Greenwich Biosciences, Inc. (#508) Boston & Virtual

Greenwich Biosciences, now part of Jazz Pharmaceuticals, is focused on discovering, developing, and commercializing novel therapeutics from its proprietary cannabinoid product platform. It is our passion and purpose to continually seek solutions that transform the lives of those living with rare and severe neurological diseases.

For additional information, please visit www.GreenwichBiosciences.com.

For more information about our product, please visit www.GBmedicine.com.



Greenwood Genetic Center (#910)

The Greenwood Genetic Center is a nonprofit institute organized to provide clinical genetic services, diagnostic laboratory testing, educational programs and resources, and research in the field of medical genetics.

Hackensack Meridian Health (#315)

Hackensack Meridian Health is a leading not-for-profit health care organization that is the largest, most comprehensive and truly integrated health care network in New Jersey, offering a complete range of medical services, innovative research and life-enhancing care aiming to serve as a national model for changing and simplifying healthcare delivery through partnerships with innovative companies and focusing on quality and safety.

Exhibitors

Invitae (#317)

Invitae's mission is to bring comprehensive genetic information into mainstream medical practice to improve the quality of healthcare for billions of people. Our goal is to aggregate the world's genetic tests into a single service with higher quality, faster turnaround time and lower prices. Visit www.invitae.com.

Labcorp (#922)

Labcorp is a leading global life sciences company that provides vital information to help doctors, hospitals, pharmaceutical companies, researchers and patients make clear and confident decisions. Through our unparalleled diagnostics and drug development capabilities, we provide insights and accelerate innovations to improve health and improve lives. Learn more about and Labcorp at www.Labcorp.com or follow us on LinkedIn and Twitter @Labcorp.

Lineagen (#424)

Lineagen, a Bionano Genomics Company, is a provider of genetic testing services focused on serving individuals with neurodevelopmental disorders. Lineagen helps make genetic testing accessible to both patients and providers by combining simple non-invasive sampling and advanced testing technology with dedicated service and support.

LivaNova (#514)

As pioneers of the VNS (Vagus Nerve Stimulation) Therapy® system, we continue to advance medical device solutions for people affected by drug-resistant epilepsy. We strive to help where it really counts, where it truly matters the most. Sharp, responsive and effective – at LivaNova we serve health and improve lives. Day by day. Life by life.

Marinus Pharmaceuticals Virtual

Marinus Pharmaceuticals is a clinical stage biopharmaceutical company committed to improving the lives of patients affected by rare seizure disorders. At Marinus, we are dedicated to the development of investigational ganaxolone, for the potential treatment of pediatric and adult patients with chronic and acute seizure disorders.

Merz Therapeutics (#815)

Merz Therapeutics addresses the unique needs of people who suffer from movement disorders, neurological conditions, and other health conditions that severely impact patients' quality of life. Our patient-centric approach, cutting-edge research and development efforts, and scientific medical affairs resources continue the advancement of new treatment standards, including botulinum toxin.

National Institute of Neurological Disorders and Stroke (NINDS) Virtual

The National Institute of Neurological Disorders and Stroke (NINDS) (www.ninds.nih.gov), part of the National Institutes of Health (NIH), provides information about research funding and free publications for patients and their families on neurological disorders. Home of the free Migraine Trainer™ app: https://www.ninds.nih.gov/sites/default/files/ninds_migraine_trainer_flyer_508c.pdf Disorder information: <https://www.ninds.nih.gov/Disorders>. NINDS publications catalog: <https://catalog.ninds.nih.gov/>.

Nationwide Children's Hospital (#323)

Nationwide Children's is ranked among the six best children's hospitals for Neurology and Neurosurgery by US News. Unique areas of focus include stroke, intracranial hypertension, spinal muscular atrophy and muscular dystrophy – including groundbreaking clinical and translational research. We are also top 10 in NIH funding among freestanding children's hospitals.

Neurelis, Inc. (#306, 313)

Boston & Virtual

Neurelis, Inc. is an innovation-driven neuroscience company focused on the development and commercialization of product candidates and innovative delivery technologies for the broader central nervous system (CNS), including epilepsy and psychiatry. In 2020, Neurelis reached a milestone in patient care with its first FDA-approved treatment. For information, please visit <http://www.neurelis.com>.

Neurotech, LLC (#916)

Accredited by the Joint Commission, Neurotech, LLC specializes in EEG services including in-home, long-term, and continuous EEG monitoring. Our EEG monitoring services improve patients' comfort and provides a cost-effective alternative to a hospital stay. Neurotech cEEG Partners, LLC provides hospitals with continuous EEG monitoring in the ICU and EMU.

GOLD SPONSOR

Novartis Gene Therapies (#709) Boston & Virtual

Novartis Gene Therapies, Inc. (formerly AveXis) is dedicated to developing and commercializing gene therapies for patients and families devastated by rare and life-threatening neurologic genetic diseases. Our initial gene therapy for spinal muscular atrophy (SMA) has been approved in more than 40 countries.



Novartis Gene Therapies – Medical (#725)

AveXis is now Novartis Gene Therapies. Novartis Gene Therapies is dedicated to developing and commercializing gene therapies for patients and families devastated by rare and life-threatening neurological genetic diseases.

Parent Project Muscular Dystrophy (#604)

Parent Project Muscular Dystrophy fights to end Duchenne. We accelerate research, raise our voices to impact policy, demand optimal care for every family, and strive to ensure access to approved therapies. Our Decode Duchenne program provides free genetic testing (diagnostic and carrier) for families living in the US or Canada.

Passage Bio (#911)

At Passage Bio, we are on a mission to provide life-transforming genetic medicines for patients with CNS diseases that replace their suffering with boundless possibility, all while building lasting relationships with the communities we serve. More information is available at www.passagebio.com.

Pediatric Search Partners (#606)

Pediatric Search Partners is a boutique search firm focused solely on pediatrics, pediatric subspecialties, and physician leaders within children's hospitals and healthcare organizations. With more than 75 years of combined experience, we deliver a proven track record of joining pediatric physicians and leaders with the hospitals and practices where they can fulfill their goals and dreams. We take care of those who take care of kids.

Pediatric Medical Group (#809)

Pediatric Medical Group, a part of the Mednax family, is a provider of maternal-fetal, newborn and pediatric subspecialty services. Starting as a single neonatology practice in 1979, we have evolved into a multi-specialty medical group – capable of delivering a women's and children's continuum of care – meeting the needs of our patients and hospital partners.

PerkinElmer Genomics (#909)

PerkinElmer Genomics is a state-of-the-art biochemical and molecular genetics laboratory that provides newborn screening and genomic testing services around the world. With over 8 million newborns screened since 1994, our laboratory pairs decades of newborn screening experience with a leading-edge clinical genomics program to offer one of the world's most comprehensive programs for detecting clinically significant genomic changes.

SILVER SPONSOR

PTC Therapeutics Inc. (#414)

Boston & Virtual

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. Our mission is to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more, please visit www.ptcbio.com.



Saint Francis Health System (#524)

Saint Francis Health System is a Catholic, not-for-profit health system wholly governed and operated in Tulsa, Oklahoma. The Hospital is a 1,112-bed tertiary center, which includes the region's only children's hospital and level IV neonatal intensive care unit, a 168-bed heart hospital and Tulsa's leading trauma and emergency center.

Sanford Health (#825)

Sanford Health, one of the largest health systems in the United States, is dedicated to the integrated delivery of health care, genomic medicine, senior care and services, global clinics, research and affordable insurance. The organization includes 46 hospitals, 1,400 physicians and more than 200 Good Samaritan Society senior care locations in 26 states and 10 countries.

Sarepta Therapeutics (#715, 814)

At Sarepta, we are working with urgency to develop breakthrough therapies to treat genetic diseases. Currently, we have more than 40 investigational therapies in various stages of development—many already in late-stage clinical trials. For more information, please visit our website at <https://www.sarepta.com/> or contact <mailto:medinfo@sarepta.com>.

Scholar Rock, Inc. (#915)

Boston & Virtual

Scholar Rock, Inc. is a clinical stage biopharmaceutical company focused on discovery and development of innovative medicines for the treatment of serious diseases. Please join us at our booth or virtually at www.scholarrock.com to learn more about Scholar Rock, our pipeline and the importance of myostatin in Spinal Muscular Atrophy.

Sentynl Therapeutics (#822)

Menkes disease is a rare X-linked recessive disorder caused by mutations in the copper transport gene, ATP7A. If untreated, many patients die before the age of three. Currently, there is no FDA-approved treatment for Menkes disease.

Stoke Therapeutics (#824)

Stoke Therapeutics is a biotechnology company dedicated to addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines. Stoke's proprietary TANGO (Targeted Augmentation of Nuclear Gene Output) approach uses antisense oligonucleotides to selectively increase protein levels. Our medicines have the potential to be disease-modifying therapies to address the genetic causes of diseases.

Theranica USA (#912)

Theranica develops non-invasive, drug-free, personalized wearable therapeutic products for the treatment of migraine, ages 12 and up, by integrating the latest neuroscience research with notable technological expertise. Theranica's smartphone-controlled prescription migraine device, Nerivio®, was recognized in TIME Magazine's 2019 annual list of the 100 Best Inventions.

Tris Pharma (#425)

Tris Pharma is a New Jersey-based specialty pharmaceutical company focused on the development and commercialization of innovative medicines that address unmet patient needs. Tris has used its innovative LiquiXR technology to develop a portfolio of differentiated solid and liquid ADHD products, which it markets in the United States. For more information, please visit www.trispharma.com.

UCB, Inc. (#423)

At UCB, we come together everyday laser-focused on a simple question: How will this create value for people living with severe diseases? We are a global biopharmaceutical company committed to innovation to improve the lives of people with neurological and immunological diseases, finding solutions to meet their unique needs.

Upsher-Smith Laboratories (#410)

Upsher-Smith Laboratories, LLC is a trusted U.S. pharmaceutical company. We offer a suite of branded central nervous system (CNS) medications to help patients living with migraine headaches and seizure disorders. For more information, visit www.upsher-smith.com.

UT Health Austin Pediatric Neurosciences at Dell Children's (#512)

UT Health Austin Pediatric Neurosciences at Dell Children's diagnoses, treats, and manages the care of children and adolescents with medical conditions of the central nervous system, including epilepsy, cerebral palsy, neuromuscular diseases, congenital abnormalities, movement disorders, headaches, genetic conditions, neurodevelopmental disorders, brain tumors, and acquired brain injuries.

Variantyx (#610)

Variantyx uses a whole genome sequencing platform to provide the most comprehensive and flexible genomic testing available, overcoming the limitations of traditional technologies. Using a single patient sample, followed by sequencing of the entire nuclear and mitochondrial DNA, our Genomic Unity testing provides analysis of small sequence changes, copy number variants, mitochondrial small sequence changes and deletions, and short tandem repeat expansions for more than 25 genes.

Xenon Pharmaceuticals Inc. (#913)

Xenon Pharmaceuticals (NASDAQ:XENE) is a clinical stage biopharmaceutical company committed to developing innovative therapeutics to improve the lives of patients with neurological disorders. We are advancing a novel product pipeline of neurology therapies to address high unmet medical needs, with a focus on epilepsy. For more information, please visit www.xenon-pharma.com.

SILVER SPONSOR

Zogenix (#309) Boston & Virtual

Zogenix is a global biopharmaceutical company committed to developing transformative therapies to improve the lives of patients and their families living with rare diseases.

ZOGENIX