ASENT 2022
ANNUAL MEETING
Virtual Neuroscience and Neurotherapeutics Conference

ADVANCE PROGRAM

Virtual Meeting
Feb 28 - Mar 3, 2022

Opening Symposium: February 28, 2022

REGISTER TODAY: https://asent2022reg.eventbrite.com
ASENT 2022 Annual Meeting

The American Society for Experimental Neurotherapeutics (ASENT) Annual Meeting offers scientific symposia featuring leading-edge research in translational neurology and neuroscience, exciting and engaging poster sessions, and pipeline presentations to keep you at the forefront of what to expect in the neurotherapeutics drug and device markets.

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About ASENT 2022

Annual Meeting Overview
ASENT 2022 is the premier neurotherapeutics conference where senior executives from leading payers, providers, employers, academic institutions, investors, fast-growing startups, pharma, policymakers, advocate organizations, funders and innovation centers in the neurology and neuroscience space gather to ask one question: how can we improve the process of bringing neurotherapeutics to market?

The plenary sessions, panel discussions, networking meetings, outstanding pipeline presentations and poster sessions will focus on the latest science in neurotherapeutics including innovations in rare disease, antisense technology across disease states, novel delivery systems, gene therapy and biomarkers, and of course the latest drug therapies and devices.

MEETING DETAILS

ASENT 2022 Meeting Dates
Monday, February 28 - Thursday, March 3, 2022

VIRTUAL FORMAT

ABSTRACT SUBMISSIONS
Open through December 13, 2022
https://www.eventbrite.com/e/
189784930837

REGISTRATION
Visit: https://asent2022reg.eventbrite.com

Meeting Location
ONLINE

WHO ATTENDS

Physician-Scientists
Neurologists
Neuroscientists
Fellows
Postdocs
Trainees
Founders
Funders
Investors
Industry Leader
NonProfit Organizations (NORD, Alzheimer’s Assoc. etc.)
Journal Editors
Drug and Device Companies
Communication Companies
Representatives from NIH, NIA, NIDA, NINDS and FDA

WHAT IS ASENT?
The American Society for Experimental Neurotherapeutics (ASENT) is an independent non-profit organization established in 1997 by leaders in academia, government, advocacy and industry to facilitate the process by which new therapies are made available to patients with neurological disorders. Its primary goal is to encourage and advance the development of novel and improved therapies for diseases and disorders of the nervous system.
### ASENT 2022 Schedule at a Glance

#### Monday, February 28, 2022

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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| 10:00 a.m. – 12:00 p.m. | Presidential Symposium  
Rare Neurological Diseases  
– presented in partnership with National Organization for Rare Disorders (NORD) |
| 12:00 p.m. – 12:15 p.m. | Break                                                                |
| 12:15 p.m – 1:30 p.m. | Concurrent Symposia  
Antisense Oligonucleotide Therapy in Rare Neurological Diseases  
Emerging Brain Lipid Pharmacology for Neurodegenerative Disorder |
| 1:30 p.m. – 2:00 p.m. | Sponsored Symposium                                                  |
| 2:00 p.m. – 3:00 p.m. | Poster Discussion                                                    |

#### Tuesday, March 1, 2022

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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| 10:00 a.m. – 11:15 a.m. | Plenary Session  
Alzheimer’s Disease After Aducanumab                                 |
| 11:15 a.m. – 11:30 a.m. | Break                                                                |
| 11:30 a.m – 12:45 p.m. | Concurrent Symposia  
Devices and Software as Therapeutics for Substance Use Disorders (SUD)  
Translational Bioinformatics in Drug Repurposing and combination therapy development for Alzheimer’s Disease |
| 12:45 p.m. – 1:00 p.m. | Break                                                                |
| 1:00 p.m. – 3:00 p.m. | Pipeline Presentations  
Emerging Neurotherapeutics Presentations                                |

#### Wednesday, March 2, 2022

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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| 10:00 a.m. – 11:15 a.m. | Plenary Session  
Emerging Science of the Exposome and Its Significance to Neurotherapeutics |
| 11:15 a.m. – 11:30 a.m. | Break                                                                |
| 11:30 a.m – 12:45 p.m. | Concurrent Symposia  
Innovative Treatments for Rare Neurodevelopmental Diseases  
The Use of Digital Monitoring Devices in Neurological Clinical Studies |
| 12:45 p.m. – 1:00 p.m. | Break                                                                |
| 1:00 p.m. – 3:00 p.m. | Pipeline Presentations  
Emerging Neurotherapeutics Presentations                                |

#### Thursday, March 3, 2022

<table>
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<th>Time</th>
<th>Event</th>
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| 10:00 a.m. – 11:30 a.m. | Plenary Session  
Covid-19 and The Brain: Update 2022                                  |
| 11:30 a.m. – 11:45 a.m. | Break                                                                |
| 11:45 a.m – 1:00 p.m. | Concurrent Symposia  
Not a One-Trick Pony: Repurposing Established Drugs for New Neurological Indications  
New Approaches to Pain Therapeutics R&D: Models and Results from Academic and Industry Sectors |
| 1:00 p.m. – 3:00 p.m. | Pipeline Presentations  
Emerging Neurotherapeutics Presentations                                |
10:00 a.m. – 12:00 p.m.  Presidential Symposium

Rare Neurological Diseases – presented in partnership with National Organization for Rare Disorders (NORD)

This Presidential Symposium in conjunction with the National Organization for Rare Disorders will focus on the challenges of Rare Neurological Disorders addressing approaches to diagnoses, development of therapeutic interventions, and regulatory issues including barriers and successes and innovative initiatives.

CHAIR: Bennett Lavenstein, MD, Children’s National Hospital
CO-CHAIR: Thomas Sutula, MD, PhD, University of Wisconsin, Madison

FACULTY

Introduction to Rare Disease Neurotherapeutics
Peter Saltonstall, National Organization of Rare Disorders

TBD
Timothy Yu, MD, PhD, Boston Children’s Hospital

TBD
Edward Neilan, MD, PhD, National Organization of Rare Disorders

TBD
Edward Kaye, MD, Stoke Therapeutics

Issues in Orphan Products Development - especially in Neurodevelopmental Diseases
Marlene Haffner, MD, MPH, Haffner Associates

Panel Discussion

12:00 p.m. – 12:15 p.m.
Break

12:15 p.m. – 1:30 p.m.  Concurrent Symposia

Antisense Oligonucleotide Therapy in Rare Neurological Diseases

FDA approval for an antisense oligonucleotide (ASO) that addresses a previously untreated disease, spinal muscular atrophy, has increased discovery and development efforts for this class of gene therapies. Three experts in ASO therapeutics will describe the application of ASO technology to the treatment of rare neurological diseases. The session will discuss recent advances in ASO delivery, toxicity, manufacturing, N=1 studies, and emphasize ASO applications to rare neurological diseases, including ALS and neuromuscular disease.

CHAIR: Bryan J. Traynor, MD, PhD, National Institutes of Health, National Institute on Aging
CO-CHAIR: C. Anthony Altar, PhD, Splice Therapeutics

FACULTY

Progress in ASO Therapeutics for Rare Diseases
Jonathan Watts, PhD, University of Massachusetts Medical School

Bioconjugate and Nanotechnology for Oligonucleotide Delivery
Matthew Wood, MD, DPhil, University of Oxford

Computational Cellular and Chemical Approaches to Reduce ASO Neurotoxicity
Peter Hagedorn, MSc, PhD, Roche Pharma

Panel Discussion
**Emerging Brain Lipid Pharmacology for Neurodegenerative Disorder**

Lipids account for up to 50% of the brain’s dry weight and comprise thousands of distinct biochemical structures whose subcellular and intracellular expression regulates many levels of neurobiology including organelle homeostasis, synaptic function, stress responses, cell death, inflammation and repair. Not surprisingly, a key role for lipid biochemistry dysregulation has been emerging in neurological disorders involving myelin repair, neuroinflammation, and neurodegeneration. Indeed, the major genetic risk factors driving Alzheimer’s disease and Parkinson’s disease, the most common neurodegenerative disorders, participate in lipid homeostasis (APOE4 and GBA respectively). Advances in genomic, proteomic and lipidomic technologies as well as translational model systems are providing new opportunities for pharmacological approaches to regulate brain lipids. Several such approaches are currently in clinical development and are taking advantage of readily available peripheral lipid biomarkers.

**CHAIR:** Dan Tardiff, PhD, Yumanity  
**CO-CHAIR:** Aditya Joshi, MD, University of Pennsylvania

**FACULTY**

- **Development of a glucocerebrosidase activator for the GBA subtype of Parkinson’s disease**  
  Peter Lansbury, PhD, Bial Biotech

- **Fatty acid metabolism controls the reparative phenotype of phagocytes in the CNS**  
  Jeroen Bogie, PhD, Hasselt University

- **Targeting APOE metabolism to reduce neurodegeneration and inflammation**  
  Jason Ulrich, PhD, Washington University at St. Louis

- **Targeting a-Synuclein Lipidopathy in Parkinson’s Disease with YTX-7739, a Brain Penetrant Stearoyl CoA Desaturase Inhibitor**  
  Dan Tardiff, PhD, Yumanity Therapeutic

**Panel Discussion**

**Poster Discussion**

ASENT has developed an interactive Poster Session experience designed to increase presenter and attendee discussion at the ASENT 2022 Annual Meeting. All posters will be displayed on the annual meeting platform with a link to the presenter profile. Posters presenters will be assigned to a discussion day and meeting attendees will have the opportunity to view posters at any point during the preview days and throughout the meeting. Poster discussions will be moderated by Program Committee Members to ensure a lively discussion.

**CHAIR:** Carolyn Tallon, PhD, Johns Hopkins University

**Live Discussion**
10:00 a.m. – 11:15 a.m.  
**Plenary Session**  
**Alzheimer’s Disease After Aducanumab**  
The goal of the symposium is to present the economic and healthcare industry implications of the controversial regulatory approval of Aducanumab, a fair-minded review of the evidence, the process, an overview of the implications for future Alzheimer’s and other neurologic drug development as well as other research areas in AD that are possible, such as Amyloid backups, Tau, and inflammation.

**CHAIR:** Andrew J. Cole, MD, FRCP(C), Harvard Medical School

**FACULTY**  
**Regulatory Focus on Alzheimer’s after Aducanumab**  
Jalayne Arias, JD, MA, University of California, San Francisco

**Research Focus on Alzheimer’s after Aducanumab**  
Karl Herrup, PhD, University of Pittsburgh School of Medicine

**Panel Discussion**

11:15 a.m. – 11:30 a.m.  
**Break**

11:30 a.m – 12:45 p.m.  
**Concurrent Symposia**  
**Devices and Software as Therapeutics for Substance Use Disorders (SUD)**  
Exacerbated by COVID-19, the opioid epidemic continues to ravage the United States. In addition to canonical pharmacologic and psychological approaches to SUD treatment, innovative device-based therapeutic solutions, including software as medical device, have recently emerged. During this symposium, the executives from some of the most disruptive and innovative device startups in today’s healthcare ecosystem will discuss the opportunities and challenges of this new field.

**CHAIR:** Stacie Gutowski, PhD, National Institute of Health, National Institute of Drug Abuse

**FACULTY**  
**Woebot for Substance Abuse Disorders**  
Athena Robinson, PhD, Woebot Health  
Maddison Pirner, MS, Woebot Health

**Software-based therapeutics: longitudinal data on impact of disease as an emerging standard of treatment**  
Yuri Maricich, MD, MBA, Pear Therapeutics

**Clinical applications of transcutaneous auricular neurostimulation for opioid use disorder**  
Navid Khodaparast, PhD, Spark Biomedical

**Panel Discussion**
Concurrent Symposia

Translational Bioinformatics in Drug Repurposing and Combination Therapy Development for Alzheimer's Disease

The emergence of biomedical big data and advances in computational technology have created unprecedented opportunities for drug repositioning and combination therapy development, particularly for CNS diseases including Alzheimer’s Disease (AD). In this symposium, you will have the opportunity to hear the most recent progress made from academic investigators, and to learn from biopharma senior scientists in sharing their drug repurposing effort from industry. The opportunities and challenges will also be discussed to help realize the full potential of drug repurposing for AD and CNS diseases.

CHAIR: Jean Yuan, PhD, National Institute of Health, National Institute on Aging
COCHAIR: Danilo Vitorovic, MD, Loma Linda University Health

FACULTY

Computational Methods and Practical Considerations in Repurposing Drugs for Neurosciences
Pankaj Agarwal, PhD, BioInfi

From Machine Learning to Basket Clinical Trial: Testing the JAK inhibitor baricitinib in Alzheimer’s disease and ALS
Mark W. Albers, PhD, Massachusetts General Hospital, Harvard Medical School

Precision Medicine and Computational Drug Repurposing for Alzheimer’s Disease
Yadong Huang, MD, PhD, University of California, San Diego

Panel Discussion

12:45 p.m. – 1:00 p.m.
Break

1:00 p.m. – 3:00 p.m.

Pipeline Presentations

The ASENT Pipeline Sessions are brief podium presentations. These presentations are derived from the abstract submissions and vetted by our abstract review committee to ensure the highest caliber and most novel research.

CHAIR: Stewart Factor, DO, Emory University
CO-CHAIR: Carolyn Tallon, PhD, Johns Hopkins University

TOPICS

The NIH HEAL Initiative/National Institute of Neurological Disorders and Stroke’s Early Phase Pain Investigation Clinical Network (EPPIC-Net): Year 2 Update


Brain Penetrant scFv Antibody Block of P2X4 Receptor for Treatment of Chronic Pain

Software-based Therapeutics: Longitudinal Data on Impact of Disease as an Emerging Standard of Treatment

Inhibition of EV Biogenesis Reduces Tau Propagation in Seeded Tau Model of Alzheimer’s Disease

Discovery of Kv7 Activators for the Treatment of Neuropathic Pain

Novel, Non-opioid, Non-addictive Intrathecal Therapy for the Treatment of Chronic Pain

Non-Addictive Analgesic Small Antibody Therapeutic Development Targeting CCKBR

**Plenary Session**

**Emerging Science of the Exposome and Its Significance to Neurotherapeutics**

The exposome is defined as all exposures a person receives during the lifespan including internal (e.g., microbiome) and external (e.g., physical-chemical, social) sources. Major progress is being made in data collection, curation, and analysis. This session will discuss the exposome concept, significance of the exposome within the neurological disease community, and scientific approaches to advance the field of exposome research and how these could be used in experimental neurotherapeutics.

CHAIR: David Jett, PhD, National Institutes of Health

FACULTY

The Exposome: A Tool for Discovery
Yuxia Cui, PhD, National Institute of Environmental Health Sciences

ALS and the Exposome
Eva L. Feldman, MD, PhD, University of Michigan

TBD
Gary W. Miller, PhD, Columbia University

Panel Discussion

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**Concurrent Symposia**

**Innovative Treatments for Rare Neurodevelopmental Diseases**

Neurodevelopmental disease-modifying drug discovery is increasing enormously, however, high unmet needs remain. This is notably true for more rare monogenetic disorders, such as Angelman, Rett, and Dravet syndromes, and across modalities (e.g. small molecule, gene therapy and antisense oligonucleotides). This symposium will discuss scientific advances, hurdles in drug development and delivery, and a fair-balanced review of advantages and disadvantages from bench to bedside.

CHAIR: Joseph Sullivan, MD, University of California San Francisco

CO-CHAIR: Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center

FACULTY

Wearing MANY hats: From Impossible to Possible: A parent’s journey through drug development
Allyson Berent, DVM, DACVIM, GeneTx Biotherapeutics

Therapeutic Antisense Oligonucleotide (ASO) Interventions for Neurodevelopmental Disorders: Lessons from Angelman Syndrome
Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center

Utility of EEG as a biomarker in clinical development: Angelman Syndrome
Rob Komorowski, PhD, Ionis Pharmaceuticals

Dravet syndrome: Unmet need for seizures and non-seizure co-morbidities
Joseph Sullivan, MD, University of California San Francisco

Developing STK-001: An Antisense Oligonucleotide (ASO) Treatment for Dravet Syndrome
Kimberly A. Parkerson, MD, Stoke Therapeutics

Disease Modifying Therapies in Neurodevelopmental Disorders (NDDs) Becoming a Reality
Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center

Panel Discussion
Concurrent Symposia

The Use of Digital Monitoring Devices in Neurological Clinical Studies
In many neurodegenerative diseases, we see the measures of quality of life, mobility, quality of sleep etc., as measurements that may reflect the stage of the disease. While QoL questionnaires rely on subjective reporting of patients, digital monitoring devices can bridge this gap by allowing for quantitative, frequent, reliable and clinically meaningful measurements of the state of patients in their daily lives. This rich dataset can help us monitor the treatment response and disease progression.

CHAIR: Sharon Tamir, Karyopharm Therapeutics
CO-CHAIR: Suhayl Dhib-Jalbut, MD, Rutgers Health

FACULTY
Deep Phenotyping Using Digital Health Technology in Parkinson's Disease
Jamie Lynn Adams, MD, University of Rochester Medical Center

Remote Digital Monitoring Technology and AI-driven Analytics in Neurodegenerative Diseases
Amir Lahav, ScD, Redenlab

Regulatory Considerations When Using Digital Health Technologies in Clinical Investigations
Christina Webber, PhD, FDA

TBD
Patrick Antkowiak, Center for Devices and Radiological Health, FDA

Panel Discussion

12:45 p.m. – 1:00 p.m.
Break

1:00 p.m. – 3:00 p.m.
Pipeline Presentations

CHAIR: Stewart Factor, DO, Emory University
CO-CHAIR: Carolyn Tallon, PhD, Johns Hopkins University

TOPICS
Fenfluramine (Fintepla®) Treatment Improves Everyday Executive Functioning in Preschool Children With Dravet Syndrome: Analyses From 2 Pooled Phase 3 Trials

Preclinical Development of NRTX-1001, an Inhibitory Interneuron Cellular Therapeutic for the Treatment of Chronic Focal Epilepsy

Interim Safety, Pharmacokinetics (PK), and Cerebral Spinal Fluid (CSF) Exposure Data from the Phase 1/2a MONARCH Study of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS)

Phase 2b Efficacy and safety of XEN1101, a novel potassium channel opener, in adults with focal onset seizures (X-TOLE)

The Impact of Disease Severity on Efficacy from a Phase 2b Study of XEN1101, a novel potassium channel opener, in adults with focal epilepsy (X-TOLE)

GM6 attenuates activated cofilin and beta-arrestin2 impact on pathological tau and decreasing tau aggregates in Alzheimer’s disease (AD) and frontotemporal lobar degeneration (FTLD)

KPT-8602: A SINE Compound for the treatment of Duchenne muscular dystrophy (DMD)

Oral small molecule hepatocyte growth factor/MET positive modulator ATH-1020 reduces depression-like behaviors and normalizes pathological EEG mismatch negativity in preclinical models

Positive modulation of hepatocyte growth factor/MET by a novel small molecule induces neurotrophic and procognitive effects

Pharmacologically Targeting Inducible Prostaglandin E Synthase to Counteract Neuroinflammation-associated Cognitive Impairments
Plenary Session

Covid-19 and The Brain: Update 2022
This plenary session features Dr. Avindra Nath, MD, PhD, of the NIH, who will address the neurological involvement and potential mechanism(s) of Covid-19 toxicity in the CNS. Our other speakers will describe brain imaging, function, and mechanism studies that reveal long-term CNS consequences of Covid-19 infection in adults and children. Speakers will present up-to-date evidence on how the virus creates CNS damage and compromises functions, as evidenced by sensory, cognitive, and behavioral impairments that last beyond the typical symptoms of Covid-19.

CHAIR: C. Anthony Altar, PhD, Splice Therapeutics

FACULTY

Pathophysiology and therapeutic targets for Neuro-COVID
Avindra Nath, MD, PhD, National Institutes of Health

Clinical features and outcome following Neuro-COVID in children
Dr. Ming Lim, Evelina London Children’s Hospital

Characterizing Post-acute Sequelae of Covid-19 (PASC): Overview and the NIH RECOVER Initiative
Clinton Wright, MD, MS, National Institutes of Health

Panel Discussion

Concurrent Symposia

Not a One-Trick Pony: Repurposing Established Drugs for New Neurological Indications
Many diseases have multifactorial pathophysiology, creating an opportunity to treat them with therapies originally developed for different indications. Here, we will discuss the development of products that utilize already established drugs from other fields of medicine for novel neurological indications.

CHAIR: Sharon Tamir, Karyopharm Therapeutics
COCHAIR: Aditya Joshi, MD, University of Pennsylvania

FACULTY

Targeting multiple neuron death pathways in neurodegenerative diseases: clinical trial experience with sodium phenylbutyrate and taurursodiol
Machelle Manuel, PhD, Amylyx

Breaking the paradigm – PrimeC as a novel approach to ALS therapy
Shiran Zimri, PhD, Neurosense

Repurposing anti-IL-6R antibody from RA to NMOSD: Challenges and successes
Takashi Yamamura, MD, PhD, National Institute of Neuroscience in Tokyo

Panel Discussion
Concurrent Symposia

New Approaches to Pain Therapeutics R&D: Models and Results from Academic and Industry Sectors

Development of novel pain therapeutics continues to present significant challenges, demonstrated by data showing only a 2% probability of drug approval for Phase I candidate pain therapeutics, compared to an overall 10% probability in other disease areas. Challenges include unknown neurobiological mechanisms of pain, translation of preclinical data, large placebo effects and disease population heterogeneity. Recently, much more emphasis has been placed on the urgent need to develop successful non-addictive therapeutics for pain as a result of efforts to address the opioid crisis. This symposium will include an overview of new models for pain therapeutics that address the challenge of pain therapeutics development from several perspectives including those representing the scientific, process management and financial challenges.

CHAIR: Mary Ann Pelleymounter, PhD, National Institutes of Health/National Institute of Neurological Disorders and Stroke
COCHAIR: Debra Ehrlich, MD, MS, National Institutes of Health/National Institute of Neurological Disorders and Stroke

FACULTY
Dual Ion channel Modulation, a Novel Mechanism for Treatment of Sensory Hyperexcitability
Haim Belinson, MSc, PhD, BSense BioTherapeutics

Leveraging A Single-Sponsor Master Protocol to Address the Challenges Inherent in Pain Therapeutic Development
Kelly Knopp, PhD, Lilly

HEAL Initiative Pain Therapeutics Development: A flexible approach for academics and small businesses
Michael Oshinsky, PhD, National Institutes of Health/National Institute of Neurological Disorders and Stroke

Panel Discussion
ASENT 2022 Registration Options

Registration is now open for the ASENT 2022 Virtual Annual Meeting! Be sure to take a moment to register and join your colleagues for this exciting event!

### REGISTRATION RATES

<table>
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<th>Reg Type</th>
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<tr>
<td>Neurotherapeutics Course Alumni</td>
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**NOTE:** We hope to be back in person in 2023. The in person meeting registration rates will be reflective of hotel, travel, food and beverage costs. Please bear this in mind as you budget for the coming year.

How to Register

**Step 1:** Visit: [https://asent2022reg.eventbrite.com](https://asent2022reg.eventbrite.com)

**Step 2:** Look for confirmation email

**Step 3:** Follow directions in confirmation email to visit annual meeting platform and complete your profile in advance of the meeting.

**Step 4:** Join the meeting on Feb 28 - Mar 3, 2022

*(don’t worry we’ll remind you)*

**Bonus:** Become a member so you have access to the recorded sessions all year

ASENT 2022 Session Recordings

All Virtual Annual Meeting registrations also include access to the ASENT 2022 Annual Meeting Sessions Recordings for a period of 7 days. Active ASENT members will have unlimited access to the recordings beyond the initial 7 day period.

ASENT Members will have the ability to watch key sessions on-demand throughout the year. They will be able to take advantage of key sessions they may have missed. Note: Specific sessions and some presentations within a session may not be available if the presenter has not granted permission to repurpose their presentation.

To learn more about ASENT membership, scroll down or contact the ASENT team at caroline@asent.org.
ASA 22 Leadership

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University of Vermont

Become an ASENT Member

Join a community of professionals in industry, government, academia and advocacy working together to bring neurotherapeutics to market.

Visit: https://members.asent.org/Membership
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https://asent2022reg.eventbrite.com