American Society for Experimental Neurotherapeutics

Program Book

19th Annual Meeting

March 15–17, 2017

Hilton Washington DC/Rockville Hotel & Executive Meeting Center
1750 Rockville Pike, Rockville, MD 20852

Vision Statement:
To advance the science of neurotherapeutics, and to improve and accelerate the development of new treatments.

www.asent.org
### Corporate Partners

**List as of March 3, 2017**

<table>
<thead>
<tr>
<th>Level</th>
<th>Amount Range</th>
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<tr>
<td><strong>Diamond</strong></td>
<td>$25,000 - $49,999</td>
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<td><strong>Platinum</strong></td>
<td>$10,000 - $24,999</td>
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<td><strong>Silver</strong></td>
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<td><strong>Bronze</strong></td>
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<tr>
<td><strong>Supporter</strong></td>
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**Supporter**

- Axovant
- Biogen
- Eisai
- Janssen
- UCB
- GT Pharmaceuticals
- Neurorine
- Novartis
- Supernus Pharmaceuticals
- Upsher-Smith

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**www.asent.org**
Greetings,

Welcome to the 19th Annual American Society of Neurotherapeutics (ASENT) Meeting at the Hilton Washington DC/Rockville Hotel and Executive Meeting Center in Rockville, Maryland.

On behalf of the Program Committee, Board of Directors, and faculty, we are delighted you are here to participate in a program that addresses many issues in neurology and therapeutics.

Our faculty hails from all aspects of neuroscience, including the FDA, NIH, pharma, clinical and translational neurology, and advocacy groups. A day-long course on Clinical Translation for brain disorders will be held March 15 and cover clinical needs, bench to patient, and trial design.

The Pipeline Session is unique to ASENT. You will hear 25 presentations about the discovery and development of new CNS therapy candidates, a not to be missed session.

Sessions on March 16 and 17 address Alzheimer’s disease, big data, neuromodulation, drug repurposing, regulatory processes for dose determination and prescribing, rapidly-acting antidepressants, pharmacogenetics, the placebo effect, and a special session on Parkinson’s disease honoring Stan Fahn, MD, Columbia University, an ASENT founder.

Other faculty include Walter Koroschetz MD, Director of NINDS; Elizer Masliah, MD, NIA/NIH; Michael Irizarry, MD, Eli Lilly; Al Sandrock, MD PhD, Boehringer Ingelheim; Jacqueline French, MD, NYU; David Standaert, MD, PhD, UAB; Ted Dawson, MD, PhD, Johns Hopkins; William Marks, MD, Verily, and many other educators and neurotherapy leaders.

We know you will find the ASENT meeting time well-spent. Take advantage of the opportunity to interact and network with your colleagues who are participating in this year’s program. We look forward to you joining ASENT.

Sincerely,

Martha Morrell, MD
President, ASENT

C. Anthony Altar, PhD
Chair and Co-Chair, Program Committee
Jeffrey S. Nye, MD, PhD

It is with a heavy heart that we announce the passing of Jeffrey Nye M.D., Ph.D. Jeff was a tremendously enthusiastic and committed member of the ASENT leadership. He chaired the Development Committee and, even in the last months of his life, continued to reach out to his extensive professional and personal network to successfully spread the word about this organization and to encourage involvement. Jeff was a delightful man—highly intelligent, incisive and decisive, generous, kind and funny. His passing is a loss not only to ASENT, but to the entire community of those working to bring neurological therapies to patients in need. In his honor, the ASENT Leadership Fund to support annual meeting scholarships to basic and clinical neuroscientists will be renamed the Jeffrey Nye M.D., Ph.D. Leadership Fund. For more information about Jeff's many professional accomplishments, please see the tribute below from his colleagues at Johnson and Johnson Innovation.

Martha Morrell, MD, ASENT President

It is with our deepest sorrow that we inform you of the death of Jeff Nye, MD, PhD, Vice President Neuroscience Innovation and Scientific Partnership Strategy at Johnson & Johnson Innovation. Jeff had been bravely managing a serious health matter for the past year, and on Monday, March 6, 2017 he passed away at home. Jeff was 57 years old. His beloved wife, Miriam Chaloff, was by his side, and his sons, Maxwell and Leo Nye, were with their father throughout his illness. Jeff was not only a brilliant neurologist and neuroscientist, but he was a highly respected academic and adjunct Associate Professor of Pharmacology at Northwestern University Feinberg School of Medicine.

It is hard to fathom the magnitude of this loss to our community. All of us who were lucky enough to have worked with Jeff know that he represented the very finest ideals in science. He was extraordinarily dedicated, passionate about his work, intelligent, and curious. Jeff always pushed forward in our joint quest to learn more and to share all that was discovered. He relentlessly questioned the status quo. His passion was instrumental to the global launch of the Johnson & Johnson Innovation Centers, and he provided astute guidance to the investment teams on behalf of the Neuroscience Therapeutic Area.

He also led the formation of many productive scientific collaborations with universities and research consortia, and truly advocated for advancement of science in academia through many sponsorships and philanthropy. Jeff previously held several roles at Janssen R&D, including Chief Medical Officer of the East Coast Research and Early Development unit, Vice President of Experimental Medicine, and Vice President of CNS Development.

While at the Early Development unit, Jeff oversaw all experimental medicine work, which ultimately led to two new drugs entering the market, and he directed truly outstanding research on other compounds, including in the areas of gastrointestinal medicine and pain. Of note, the early development work for INVOKANA® (canagliflozin, SGLT2 inhibitor) was done under Jeff's direction. His work during this time also led to successful out-licensing strategies and results.

Prior to joining Janssen, he served as a Director of CNS Discovery at Pharmacia. Before joining the industry, Jeff was a tenured Associate Professor of Molecular Pharmacology, Biological Chemistry, and Pediatrics (Neurology) at Northwestern University. His professional life was marked by tireless service to the larger scientific community, by a desire to ask and answer important scientific questions and, above all, to make a real difference with his research. He was, not surprisingly, the recipient of numerous awards recognizing not only his scientific acumen but his dedication to improving the health of people worldwide. Jeff will be missed most acutely by his loving family but also by his many admiring colleagues, both here at Janssen and at Johnson & Johnson and in the larger neuroscience community.

We have lost an important and beloved family member. We will all work hard to commemorate the legacy of his life and research in a meaningful manner, most especially by continuing our own research into reducing the burden of these devastating illnesses, a cause that meant so much to him.

All of us who knew Jeff appreciated his extraordinary intellect, boundless energy, and focused quest to move the science forward to help people all over the world be healthier. We will all miss him very much.

Jeff’s funeral was held Thursday, March 9 at 10 am at Congregation Beth El, 375 Stony Hill Road, Yardley, PA 19067.

Donations in Jeff's honor can be made to The Fund for Johns Hopkins Medicine, Solomon H. Snyder Research Fund, 750 East Pratt Street, 17th Floor, Baltimore, MD 21202.

Please note that the Johnson & Johnson Employee Assistance Program (1-800-471-1900) offers counseling for those who may want to talk with a mental health professional about the great loss of our esteemed colleague and friend, Jeff Nye.

Sincerely,

Bill Hait
Husseini Manji
Robert Urban
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General Session and Concurrent Symposia - Plaza II&III
Clinical Translation Course and Concurrent Symposia - Plaza I
Meals and Poster Presenters - Roosevelt/Madison
Publications Committee Meeting - Twinbrook
ASENT Board Meeting and Dinner - Montrose
ASENT Patient Advocacy Council - Wilson
Membership Committee Meeting - Monroe
Communications Committee Meeting - Wilson
Corporate Planning and Development Council Lunch - Wilson
ASENT Annual Meeting Committee - Wilson
<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Location</th>
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<tbody>
<tr>
<td><strong>Tuesday, March 14, 2017</strong></td>
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<tr>
<td>5:30 am – 8:00 pm</td>
<td>ASENT Board Meeting &amp; Dinner</td>
<td>Montrose</td>
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<tr>
<td>5:30 pm – 8:00 pm</td>
<td>Publication Committee Meeting</td>
<td>Twinbrook</td>
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<tr>
<td><strong>Wednesday, March 15, 2017</strong></td>
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<tr>
<td>7:30 am – 8:30 am</td>
<td>Coffee &amp; Light Breakfast</td>
<td>Plaza Foyer</td>
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<td>8:30 am – 9:00 am</td>
<td>President’s Welcome</td>
<td>Plaza II &amp; III</td>
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<tr>
<td>9:00 am – 12:00 pm</td>
<td>Pipeline Presentations Part 1</td>
<td>Plaza II &amp; III</td>
</tr>
<tr>
<td>9:00 am – 12:00 pm</td>
<td><em>ALL DAY</em> Course in Translational Research</td>
<td>Plaza I</td>
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<tr>
<td>12:00 pm – 2:00 pm</td>
<td>ASENT Patient Advocacy Council Lunch Meeting</td>
<td>Wilson</td>
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<tr>
<td>2:00 pm – 4:00 pm</td>
<td>Pipeline Presentations Continue Part 2</td>
<td>Plaza II &amp; III</td>
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<tr>
<td>3:30 pm – 3:45 pm</td>
<td>Break</td>
<td>Plaza Foyer</td>
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<tr>
<td>4:00 pm – 6:00 pm</td>
<td>Poster Viewing</td>
<td>Plaza Foyer</td>
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<tr>
<td>5:00 pm – 6:00 pm</td>
<td>Opening Reception (all)</td>
<td>Patio Foyer and Madison</td>
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<tr>
<td>6:00 pm – 8:30 pm</td>
<td>Careers in Neuroscience Dinner (all)</td>
<td>Roosevelt/Madison</td>
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<tr>
<td><strong>Thursday, March 16, 2017</strong></td>
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<tr>
<td>6:45 am – 7:30 am</td>
<td>ASENT Committee Meeting Breakfats</td>
<td>Presidential Foyer</td>
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<td>7:00 am – 8:00 am</td>
<td>Membership Committee Meeting</td>
<td>Monroe</td>
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<td>7:00 am – 8:00 am</td>
<td>Communications Committee Meeting</td>
<td>Truman</td>
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<tr>
<td>7:30 am – 8:30 am</td>
<td>Coffee and Breakfast</td>
<td>Plaza Foyer</td>
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<tr>
<td>8:00 am – 8:30 am</td>
<td>President’s Announcements</td>
<td>Plaza II &amp; III</td>
</tr>
</tbody>
</table>

**Program Schedule**
## Program Schedule

### Thursday, March 16, 2017 continued

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
</tr>
</thead>
</table>
| 8:30 am – 12:00 pm | Opening Plenary: Alzheimer Disease: Therapeutic Horizon or Mirage  
Robert W. Hamill, MD, University of Vermont (Co-Chair)  
Steven T. DeKosky MD, McKnight Brain Institute (Co-Chair)  
- Challenges in Delaying / Preventing Alzheimer Disease  
  Walter Koroshetz, MD, NINDS  
- Challenging Issues in Developing Disease Modifying Therapy in Alzheimer Disease  
  Eric R. Siemers, MD, Eli Lilly and Company  
- Biomarkers in Alzheimer Disease  
  Leslie M. Shaw, PhD, University of Pennsylvania  
  - Expanding the Dimensions of AD Research: New Targets and Goals  
    Eliezer Masliah, MD, National Institutes of Aging, NIH  
  - Patient Participation in Trial Design: Recruitment and Retention of an “Invested” Population  
    Mary Sano, PhD, Icahn School of Medicine at Mount Sinai  
  - Challenges of and Strategies for, Testing the Amyloid Hypothesis in Alzheimer Disease  
    Alfred W. Sandrock Jr., MD, PhD, Biogen Inc. |
| 10:30 am – 10:45 am | Break  
Plaza Foyer |
| 12:00 pm – 1:30 pm | Lunch with Posters and Authors  
Roosevelt/Madison |
| 12:00 pm – 1:30 pm | Corporate Planning and Development Council Lunch  
Wilson |
| 1:30 pm – 3:00 pm | Plenary session – Hot Topics: Big Data and Neurotherapeutics  
William Marks Jr., MD, Verily (Chair)  
- Application of Big Data to Neurotherapeutics  
  William Marks, MD, Verily  
- Aggregating Data from Patient Networks: Understanding the Patient Experience  
  Paul Cannon, PhD, 23andMe  
  - Large Scale Modeling: Identifying Neural Connections and Circuits  
    Costas Anastassiou PhD, Allen Institute for Brain Science |
| 3:00 pm – 3:15 pm | Break  
Plaza Foyer |
| 3:15 pm – 5:15 pm | Plenary Session: Pharmacogenomics for CNS Medication Discovery, Development and Prescribing  
C. Anthony Altar, PhD, NeuroDrug Consulting (Chair)  
- Research, Development, and Application of a SNP-based Psychiatric Pharmacogenomic Product: Clinical and Economic Benefits  
  C. Anthony Altar, PhD, NeuroDrug Consulting  
- Genetic Enrichment Strategy in an Alzheimer’s Disease Prevention:  
  Scott Sundseth, PhD, Cabernet Pharmaceuticals  
  - Using Pharmacogenomics for CNS Target Identification  
    Todd Lencz, PhD, Feinstein Institute for Medical Research  
  - Future Innovations to the Use of Genetics in Prescribing CNS Medication  
    Falk W. Lohoff, MD, NIAAA |
| 6:00 pm – 8:30 pm | Dinner Symposium - Art, Music, Poetry and the Brain  
Victor Krauthamer, PhD, FDA (Chair)  
Dr. Jose “Pepe” Contreras-Vidal, University of Houston |

### Friday, March 17, 2017

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
</tr>
</thead>
</table>
| 6:45 am – 8:15 am | ASSENT Annual Meeting Committee  
Wilson |
| 7:30 am – 8:30 am | Coffee & Breakfast  
Presidential Foyer |
| 8:00 am – 5:00 pm | Registration  
Plaza Foyer |
| 8:30 am – 9:00 am | ASSENT Business Meeting  
Plaza II & III |
### Program Schedule

**Friday, March 17, 2017 continued**

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Location</th>
<th>Details</th>
</tr>
</thead>
</table>
| 9:00 am – 12:00 pm | Concurrent Symposium: Neuromodulation Update: Implantable Devices to Treat Disease/Disorders of the Central and Autonomic Nervous Systems | Plaza II & III    | Mike Wolfson, PhD, NIH (Co-Chair)  
Kip Ludwig, PhD, Mayo Clinic (Co-Chair)  
- Neuroumodulation at the FDA: Communication Across Divisions and New Developments  
  Carlos Peña, PhD, FDA  
- Bioelectronic Medicines – Injectable Wireless Systems for Autonomic Nervous System Neuromodulation  
  Roy Katsa, PhD, Glaxo Smith Kline  
- The Stentrode – A Minimally Invasive Option for Neuromodulation Therapies in the Brain  
  Tom Oxley, MD, PhD, Mount Sinai Health System  
- Tapping the Spinal Cord, Rootlets, and Ganglia  
  Doug Webber, PhD, DARPA |
| Break              |                                                                        | Plaza Foyer       | 10:30 am - 10:45 am  
Carlos Peña, PhD, FDA  
Tom Oxley, MD, PhD, Mount Sinai Health System  
Doug Webber, PhD, DARPA |
| 9:00 am – 12:00 pm | Concurrent Symposium: Pharmacologic Innovations and Drug Repurposing | Plaza I           | Karoly Nikolich, PhD, Alkahest (Co-Chair)  
Kathleen Clarence-Smith, MD, PhD, Chase Pharmaceuticals (Co-Chair)  
- Use of Meta-Structures for Leads on Repurposing Drugs for New Indications  
  Robert Konrat, PhD, University of Vienna  
- Regulatory Pathway for Repurposed Drugs  
  Kurt R. Karst, Hyman, Phelps & McNamara, P.C.  
- CPC-201 for Alzheimer’s Disease  
  Kathleen Clarence-Smith, MD, PhD, Chase Pharmaceuticals  
- Repurposing in Pharmaceutical Research and Development  
  Frank Dullweber, PhD, Boehringer Ingelheim  
- Nilotinib Effects in Parkinson’s Disease & Dementia w/Lewy Bodies  
  Charbel Moussa, MD, PhD, Georgetown University  
- Novel Therapeutic Against Nerve Agent-Induced Seizures and Brain Damage  
  Maria Braga, DDS, PhD, Uniformed Services University |
| 12:00 pm – 1:30 pm | Lunch with Posters and Authors                                          | Roosevelt & Madison |                                                                                                                                         |
| 1:30 pm – 3:00 pm  | Concurrent Symposium – Hot Topics: Neurodegeneration Parkinson’s Disease in 2017 | Plaza II & III    | Stanley Fahn, MD, Honoree, New York Presbyterian (Co-Chair)  
Bennett Lavenstein, MD, Children’s National Health System (Co-Chair)  
- Introduction  
  Stanley Fahn, MD, New York Presbyterian  
- Molecular Genetics Targets Therapeutics in Parkinson’s Disease  
  Ted Dawson, MD, PhD, John Hopkins Hospital  
- Neuromodulatory Advances in Parkinson’s Disease  
  Christopher Hess, MD, University of Florida |
| 1:30 pm – 3:00 pm  | Concurrent Session – Hot Topics: Therapeutics in Psychiatry and Rapidly Acting Antidepressants | Plaza I           | Christopher C. Gallen, MD, PhD, Wex Pharmaceuticals (Chair)  
- 4-Hydroxynorketamine, and AMPA Receptor Agonist is the Active Agent Producing Rapid Antidepressant Responses to Ketamine  
  Todd Gould, MD, University of Maryland School of Medicine  
- Clinical Trial Designs for Rapidly-Acting Antidepressants including Ketamine Analogues  
  Suresh Durgam, PhD, Allergan |
| 3:00 pm – 3:15 pm  | Break                                                                  | Plaza Foyer       |                                                                                                                                         |
| 3:15 pm – 5:00 pm  | Closing Plenary: The Placebo Effect: Causes and Corrections for Clinical Trials | Plaza II & III    | Amy Chappell, MD, Independent Expert Consultant (Chair)  
- The Biological Basis of the Placebo Response  
  Howard Fields, MD, PhD, UCSF  
- Placebo Response from a Regulatory Perspective  
  Josephine Briggs, MD, NCCIM  
- The Placebo Response in Psychiatry  
  William Potter, MD, PhD, NIH  
- The Placebo Response in Epilepsy  
  Jacqueline French, MD, NYU Langone Medical Center |
## Program Agenda

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
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<tbody>
<tr>
<td>9:00 am - 9:05 am</td>
<td>Overview&lt;br&gt;Stewart Factor, MD, Emory University&lt;br&gt;The Healthcare Innovation Cycle and Lessons from CIMIT&lt;br&gt;Steve Schachter, MD, Harvard Medical School</td>
</tr>
<tr>
<td>9:05 am – 9:35 am</td>
<td>Defining Clinical Needs and the Value Proposition&lt;br&gt;Mike Dempsey, CIMIT</td>
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<tr>
<td>9:35 am – 10:05 am</td>
<td>Intellectual Property&lt;br&gt;Ron Cohen, MD, Acorda Therapeutics</td>
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<tr>
<td>10:05 am – 10:35 am</td>
<td>Break</td>
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<tr>
<td>10:35 am – 11:05 am</td>
<td>Identifying Stakeholders in the Healthcare Ecosystem&lt;br&gt;Gold, PPD Global Product Development</td>
</tr>
<tr>
<td>11:05 am – 11:35 am</td>
<td>Putting Together an Action Plan from Bench to Patient&lt;br&gt;Mike Dempsey, CIMIT</td>
</tr>
<tr>
<td>11:35 am – 12:00 pm</td>
<td>Panel Discussion-All</td>
</tr>
<tr>
<td>12:00 pm – 1:00 pm</td>
<td>Lunch Break</td>
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<tr>
<td>1:00 pm – 1:30 pm</td>
<td>“Killer” Questions and Experiments&lt;br&gt;John Collins, PhD, CIMIT</td>
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<tr>
<td>1:30 pm – 2:00 pm</td>
<td>Clinical Trial Design&lt;br&gt;Karl Kieburtz, MD, University of Rochester</td>
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<tr>
<td>2:00 pm – 2:30 pm</td>
<td>NeuroNEXT Overview and Biostatistics&lt;br&gt;Christopher Coffey, PhD, University of Iowa</td>
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<tr>
<td>2:30 pm – 2:45 pm</td>
<td>Panel Discussion-All</td>
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<tr>
<td>3:00 pm – 3:30 pm</td>
<td>Strategic Partnerships&lt;br&gt;Ron Cohen, MD</td>
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<tr>
<td>3:30 pm – 3:45 pm</td>
<td>Break</td>
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<tr>
<td>3:30 pm – 4:00 pm</td>
<td>Regulatory Pathways&lt;br&gt;Carlos Peña, PhD, FDA</td>
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<tr>
<td>4:00 pm – 4:30 pm</td>
<td>SBIR and Other NINDS Funding Opportunities and Resources for Translational Researchers&lt;br&gt;Amir Tamiz, PhD, NINDS/NIH</td>
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<tr>
<td>4:30 pm – 4:50 pm</td>
<td>Panel Discussion-All</td>
</tr>
<tr>
<td>4:50 pm – 5:00 pm</td>
<td>Summary and Wrap-Up&lt;br&gt;Stewart Factor, MD, Emory University</td>
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</tbody>
</table>
Friday
March 17, 2017
Hilton Washington DC/Rockville
Hotel & Executive Meeting Center
Plaza II & III
8:30 am – 9:00 am

I. Call to Order
II. Support Recognition
III. Financial Report
III. 2017 Executive and Board Member Recognition
IV. Neurotherapeutics® Journal Report
V. Membership Report
VI. Old Business
VII. New Business
VIII. Adjournment
Save the Date – 2018

American Society for Experimental Neurotherapeutics

ASENT 20th Annual Meeting
March 7-9, 2018
Hilton Washington-DC/Rockville Hotel & Executive Meeting Center
1750 Rockville Pike, Rockville, MD 20852
Corporate Partner

Diamond Level

[Image with logos of Janssen and Johnson & Johnson]
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Supporters

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- Neurocrine Biosciences
- Novartis

Silver Level

- Supernus Pharmaceuticals
- Upsher-Smith

Partners in Health Since 1919
NEWS RELEASE

Nevro to Report Operating Results for the Fourth Quarter and Full Year 2015

2/9/2016

REDWOOD CITY, Calif., Feb. 9, 2016 /PRNewswire/ - Nevro Corp. (NYSE: NVRO), a global medical device company that is providing innovative evidence-based solutions for the treatment of chronic pain, today announced that it will release financial results for the fourth quarter and full year of 2015 after market close on Monday, February 29, 2016. Company management will host a conference call beginning at 1:30 p.m. Pacific Time (4:30 p.m. Eastern Time) on Monday, February 29, 2016.

Investors interested in listening to the conference call may do so by dialing (877) 201-0168 for U.S. callers, or (647) 788-4901 for international callers (Conference ID: 46877325), or from the webcast on the “Investors” section of the Company’s website at: www.nevro.com. Following the call, a replay will be available on the Investor Events and Presentations webpage of the Company’s website.

About Nevro

Headquartered in Redwood City, California, Nevro is a global medical device company focused on providing innovative products that improve the quality of life of patients suffering from debilitating chronic pain. Nevro has developed and commercialized the Senza spinal cord stimulation (SCS) system, an evidence-based neuromodulation platform for the treatment of chronic pain. The Senza system is the only SCS system that delivers Nevro’s proprietary HF10 therapy. Senza, HF10, Nevro and the Nevro logo are trademarks of Nevro.

Investor Relations Contact:
Nevro Investor Relations
Katherine Bock
(650) 433-3247
ir@nevro.com
Supporters

Supporter

AXOVANT
Dementia Solutions®
Pipeline Presentations

Morning Session

Introduction
Kathleen Clarence-Smith, MD, PhD; Roger Porter, MD

Neuroprotective Methylthiazole Derivatives Rescue Learning & Memory
Doug Cowart, PhD; Doris F. Cully; Gregory Thatcher

High Dose Biotin for Multiple Sclerosis
F. Sedal, MD, PhD

Orally – Active, Dual-Mechanism, Functional Selectivity CNS Ligands as Prospective Alzheimer’s Disease-Modifying Therapeutics
John A. Schetz, PhD

Phase II Study to Assess Safety and Target Engagement of AMX0035 in Alzheimer’s Disease
Kent Leslie

Stereopure Nucleic Acid Therapies in Development for the Treatment of Genetic Neurological Diseases
Michael Panzara, MD, MPH

HTT-AS suppression of HTT: A Potential Therapeutic Approach for Huntington’s Disease
Pan Li, PhD

RNA trans-splicing: A gene Reprograming Technology with Major Advantages over CRISPR for the Development of Neurotherapeutics
Loyd Mitchell, MD

Overview of the TAU (temporary authorization of use) Process that allows accelerated access to the market in France for products meeting a major medical need
Frederic Sedel, MD, PhD

Nanodendrimer Therapy for Adrenoleukodystrophy
Ali Fatemi, MD, PhD

Breaking the Code of Fragile X Syndrome – Pathophysiology, Biomarkers, and Therapeutic(s)
Hao Chen, PhD

Lisuride SC Continuous Infusion for Parkinson’s Disease
Reinhard Horowski, MD

Novel treatment of Chronic Pain
Gerhard Koenig, PhD

Drug Chess - Thinking a Few Moves Ahead: Explore a series of neurology landscape maps with a life sciences investor to see promising R&D programs in the context of both their competition as well as complementary innovation. To succeed, large companies must work with small ones to develop multiple therapeutic approaches that anticipate all possible moves on the board.

Peter Kolchinsky, PhD
Pipeline Presentations

Afternoon Session

Aβ oligomer displacement and clearance into the CSF as a novel disease-modifying strategy for Alzheimer’s disease: discovery and clinical development of CT1812
S.M. Catalano, PhD

Pairnomix: Personalized Genetic Evaluations
Matthew Fox

Saffron Crocins for Prevention of and Rescue from Retinal Neurodegeneration
John Paul Sangiovanni, ScD; Silvia Bisti, PhD; Rita Maccarone, PhD; Stefano De Marco, PhD; John M. Davis, MD; Marco Piccardi, MD; Benedetto Falsini, MD

Magnetically Enhanced Diffusion of TPA for the Treatment of Stroke
Francis (Duke) M. Creighton, PhD

Syringe-injectable Mesh Electronics: Stable Long-term Brain Electrophysiology for Neurotherapeutics and Neuroprosthesis
Tian-Ming Fu

Regulatory trends in regenerative medicine in Japan
Dr. Ken Sakushima

Defent™ ADTF Patch: Deterring Opioid Misuse & Abuse
Steve Damon

Sativex Associated With Behavioral-Relapse Prevention Strategy as Treatment For Cannabis Dependence: A Pilot Randomized Clinical Trial
Jose Trigo, PhD

Development of a Recombinant Molecular Vehicle for Intra-Neuronal Delivery of Antibody Fusion Proteins
Phillip Band, PhD

Regulatory Innovations in Neurological Disorder Therapies, Including Spinraza and Eteplirsen
F. Sasinowski, MS, MPH, JD

Concluding Remarks
M. Maral Mouradian, MD, Amir Tamiz PhD
10. Extrapolating anti-epileptic drug efficacy data from adults to children; Results from the PEACE initiative
   O’Neill D’Cruz, MD, MBA; John Pellock

   Ying Peng, MD, PhD; Yatao Yin, MD, PhD

12. Oral administration of erythromycin decreases RNA toxicity in myotonic dystrophy
   Hideki Mochizuki, MD, PhD; Masayuki Nakamori; Krzysztof Sobczak

13. Inhibiting the Proliferation of Patient-Derived Glioblastoma Multiforme (GBM) Cells and U-87 MG Cell Line Using Leaf Extract of Bacopa monnieri
   Tejas Athni, Student (11th Grade); Brajesh Vaidya; Nirmal Joshee, PhD; Prahlad Parajuli, PhD

14. TAK-063, a novel phosphodiesterase 10A inhibitor, protects striatal neurons from degeneration and ameliorates behavioral deficits in the R6/2 mouse model of Huntington’s disease
   Akina Harada; Kazunori Suzuki; Haruhide Kimura

15. Adverse Event Reporting in Clinical Trials: Time for Change
   Christopher Kenney, MD; Karl D. Kieburz; C. Warren Olanow; Stephen Bandak

16. Nalbuphine Efficacy for L-Dopa Induced Dyskinesia in Parkinson’s Disease
   M. Moral Mouradian, MD

17. HTT-AS suppression of HTT: a potential therapeutic approach for Huntington’s disease
   Pan P. Li, PhD; Ying Song; Xin Sun; Wang Zheng; Wenjuan Ye, BS; Xin Hu, PhD; Marc Ferrer, PhD; Dobrina D. Rudnicki, PhD; Russell L. Margolis, MD

18. Strategy to Improve Clinical Development
   Harumasa Nakamura, MD; Shin’ichi Takeda

19. Nanodendrimer Therapy for adrenoleukodystrophy and other genetic disorders of myelination
   Ali Fatemi, MD, MBA; Christina Mertz; Bela Turk; Kannan Rangaramanujam; Suatha Kannan

20. Migraine Prophylaxis using a Novel Caloric Vestibular Stimulation Device Migraine Prophylaxis using a Novel Caloric Vestibular Stimulation Device
   Robert Black, PhD

21. Ethoxyquin and its novel analogue are effective in multiple models of chemotherapy induced peripheral neuropathies
   Ahmet Hoke, MD, PhD; Aysel Fisgun; Nicole Reed; Jing Zhu

22. Syringe-injectable Mesh Electronics: Stable Long-term Brain Electrophysiology for Neurotherapeutics and Neuroprosthesis
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27. Preliminary Evidence for the Use of Triple Reuptake Inhibitors (TRIs) for the treatment of PTSD
Walter Piskorski, MBA; Shane A. Perrine; Frank P. Bymaster; Alok K. Dutta

28. Network for Excellence in Neuroscience Clinical Trials, NeuroNEXT: Success and Opportunities for Future Collaboration
Marianne Chase, BA; Dixie Ecklund; Michael Bosch; Brenda Thornell; Christopher S. Coffey, PhD
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29. A Novel Neurometabolic Approach to Treating Alzheimer’s Disease. Final Results of a Phase 2a Study of T3D-959 in Mild to Moderate Alzheimer’s Subjects
John Didsbury, PhD; Stanley Chamberlain; Warren Strittmatter; Hoda Gabriel

30. Lisuride - a strong dopamine (DA) agonist for Continuous Dopaminergic Stimulation (CDS) in Parkinson’s Disease (PD)
Reinhard Horowski, MD

31. A Neural Bypass Technology to Reconnect the Brain to the Body
Jeanne Greathouse, PhD; Nick Annetta; David Friedenberg; Marcie Bockbrader; W. Mysiw; Ali Rezai; Chad Bouton; Herb Bresler

32. An Overview of Two Late-Stage Studies of Intepirdine, a Novel 5HT6 Receptor Antagonist, for the Treatment of Alzheimer’s Disease and Dementia with Lewy Bodies: MINDSET and HEADWAY-DLB
Shankar Ramaswamy, MD; Lawrence Friedhoff; Ilise Lombardo; Nicholas France; Ebenezer Asare, MD
Heather Dworak, PhD; Geetha Ramaswamy, MD; Yi Mo, PhD

33. Neuroprotective Methylthiazole Derivatives Rescue Learning & Memory
Douglas Cowart, PharmD; Doris F. Cully; Gregory Thatcher

34. Sativex Associated With Behavioral-Relapse Prevention Strategy as Treatment For Cannabis Dependence: A Pilot Randomized Clinical Trial.
Jose M. Trigo, PhD; Alexandra Soliman; Lena Quilty; Benedikt Fischer; Jurgen Rehm, Peter Selby, Allan J. Barnes, Marilyn A. Huestis, Tony George, David Streiner, Gregory Staios, Bernard Le Foll

35. Improved Thrombolysis via Magnetically Conjugated Delivery cpd
Francis M. “Duke” Creighton, PhD; Colin Derdeyn, MD

36. Secretory Products of B Cells From Patients With Multiple Sclerosis Mediate Death Of Oligodendrocytes and Neurons Secretory Products of B Cells From Patients With Multiple Sclerosis Mediate Death Of Oligodendrocytes and Neurons Secretory Products of B Cell
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37. NLY001, a potential disease modifying agent for neurodegenerative diseases
Seulki Lee, PhD; Seung Pil Yun; Tae-In Kam; Hanseok Ko; Ted Dawson, MD, PhD

38. RNA trans-splicing: A gene reprograming technology with major advantages over CRISPR for the development of neurotherapeutics
Lloyd G. Mitchell, MD

39. Stereopure Nucleic Acid Therapies in Development for the Treatment of Genetic Neurological Diseases
Michael Panzara, MD, MPH
40. FLX-787, a Dual Activator of TRPV1 and TRPA1 Ion Channels, Reduces Muscle Cramp Frequency and Pain Associated with Night Leg Cramps
   **Glenn Short, PhD;** Jian Liu; Mark Versavel; Brooke Hegarty; Jennifer Szegda; Christoph Westphal, MD, PhD; Thomas Wessel, MD, PhD

41. Transdermal Fentanyl Abuse and Misuse Deterrence - Defent(TM)
   **Steven Damon;** David Enscore; Alan Smith

42. The Benefits of Trans-Resveratrol with Polyphenols in Parkinson’s Disease
   **Elizabeth K. Barber, PhD**

43. High-Throughput Screening with Repurposed Medicines Against an SCN8A Mutation Causative for Epilepsy
   **Chani M Maher, PhD;** Bryant C. Gay; Gregory R. Stewart; Aaron C. Gerlach; JulieAnn Rader, Brett M. Antonio, Sonia Santos, MSc, Karen Padilla, MT, Orrin Devinsky, MD, Matt Migh, PhD, Steven Petrou PhD, David B. Goldstein, PhD

44. Cannabidiol (CBD) reduces convulsive seizure frequency in Dravet syndrome: results of a multi-center, randomized, double-blind, placebo-controlled trial (GWPCARE1)
   **Orrin Devinsky, MD;** J. Helen Cross; Linda Laux; Eric Marsh; Ian Miller, MD; Rima Nabbout, MD; Ingrid E Scheffer, MD, PhD; Elizabeth A. Thiele, MD; Stephen Wright, MD

45. Treatment with cannabidiol (CBD) significantly reduces drop seizure frequency in Lennox Gastaut syndrome (LGS): Results of a multi-center, randomized, double blind, placebo controlled trial (GWPCARE4)
   **Elizabeth A. Thiele, MD;** Maria Mazurkiewicz-Beldzinska; Selim Benbadis; Eric D. Marsh; Charuta Joshi, MD; Jacqueline A. French, MD; Claire Roberts, PhD, Adam Taylor, PhD; Kenneth Sommerville, MD

46. High Dose Biotin for Not-active Progressive Multiple Sclerosis
   **Frederic Sedel, MD, PhD**

47. Phase II Study to Assess Safety and Target Engagement of AMX0035, a Novel Combination Therapy, in Alzheimer’s Disease
   **Kent Leslie, MS;** Steven E. Arnold; Joshua Cohen; Justin Klee

48. Saffron Crocins for Prevention of and Rescue from Retinal Neurodegeneration
   **John Paul SanGiovanni, ScD;** Silvia Bisti; Rita Maccarone; Stefano De Marco; John M. Davis, MD; Marco Piccardi, MD, Benedetto Falsni, MD

49. Orally-active, dual-mechanism, functional selectivity CNS ligands as prospective Alzheimer’s disease-modifying therapeutics
   **Dhwanil A. Dalwadi, PhD;** Aurélie Joly-Amado; Satheesh B. Ravula; Fabio C. Tucci; Marcia Gordon; John A. Schetz

50. Molecular Vehicle for Intra-Neuronal Delivery of Antibody Fusion Proteins
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Stanley Fahn, MD, is the H. Houston Merritt Professor of Neurology and Director of the Center for Parkinson's Disease and Other Movement Disorders at Columbia University in New York City. He is the Scientific Director of the Parkinson's Disease Foundation and served as President of the American Academy of Neurology (AAN) from 2001 to 2003. He works on clinical and experimental therapeutics of movement disorders, including Parkinson disease (PD).

In 1985, he co-founded The Movement Disorder Society and was elected its first president. He was the founding co-editor of the journal Movement Disorders and served in this capacity for the first 10 years of the journal's existence, until 1996. He has also served as associate editor of Neurology for 10 years. Dr. Fahn has twice served as chairman of the Advisory Committee on Peripheral and Central Nervous System Drugs for the Food and Drug Administration. He is a member of the Institute of Medicine of the US National Academies.

With Dr. Ira Shoulson, Dr. Fahn co-founded in 1986 the Parkinson Study Group (PSG), a consortium of clinical investigators dedicated to conducting controlled clinical trials on the prevention and treatment of PD. Dr. Fahn was elected and re-elected as co-chair of its Executive Committee and served for 20 years in that capacity.

Dr. Fahn was selected to be chairman and chief organizer of the first two World Parkinson Congresses that were held in 2006 and 2010. He is currently co-organizing the third World Parkinson Congress, to be held in Montreal, in October 2013.

The AAN honored Dr. Fahn with the Wartenberg Award for outstanding clinical research in 1986, the first Movement Disorder Prize for outstanding contributions in this field in 1997, and their A. B. Baker Award for outstanding educator in neurology in 1996. The American Neurological Association awarded him the first Soriano Lectureship for excellence in research. In 2007, he received the James Parkinson Medal, awarded once every 10 years by the Parkinson's Disease Foundation. Dr. Fahn and Drs. Joseph Jankovic and Mark Hallett are co-authors of the textbook, *Principles and Practice of Movement Disorders*, whose second edition was published in August 2011.
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ASENT Leaders,

I'm inviting you to provide an inaugural contribution to the Jeffrey S. Nye Leadership Fund. The purpose of the fund is to provide support to attend the ASENT Annual Meeting to trainees in fields related to neurotherapeutics. These attendees will be the future members, and ultimately leaders, of our society.

About 10 years ago my friend, Roger Porter, told me that I should go to an ASENT meeting. I said, “What is ASENT?” He told me that I would meet representatives of academia, industry, advocacy, FDA, NIH, DOD and the investment community who shared a commitment to neurotherapeutic solutions. My schedule was filled with general neurology and epilepsy subspecialty meetings, and I wasn't sure that I was interested in a meeting with a broad range of topics and attendees. But I knew never to turn down a suggestion by Roger, so I went. And it was and remains my favorite meeting.

ASENT helped me realize how big this neurotherapeutics job is - and the many talents and areas of expertise needed to get it done. ASENT put names and faces to titles and organizations and I made connections that have enriched me personally and professionally. The broad content of the meeting turned out to be very relevant to my own work. My experience with ASENT helped with my own transition from full time academia to an academic/industry hybrid position and contributed to my professional effectiveness and satisfaction.

I want to personally thank the founders of ASENT who had the foresight and talent to create this organization. I want to challenge our current leadership to be part of ASENT’s future through the Jeffrey S. Nye Leadership Fund. The Leadership Fund will provide travel/lodging support to residents, fellows, and graduate students who would otherwise be unable to attend the ASENT Annual Meeting. The scholarship is granted on a competitive basis and the numbers of scholarships depend on funds available; $2000 will cover one trainee. Information about this opportunity was sent to Neurology Chairs and Residency Training Directors. You are also welcome to make your own nominations.

Any size contribution is welcomed, all donations are tax-deductible (ASENT is a 501C-3 corporation), and all contributors will be acknowledged in the Annual Meeting program.

To make a contribution to the ASENT Leadership Fund, please send a check to: ASENT, 342 North Main Street, Suite 301, West Hartford, CT 06117. Please note “Jeffrey S. Nye Leadership Fund” in the memo of your check. If you would like to contribute by credit card, visit asent.org to donate online you will be required to sign in to your myASENT.

Thanks so much for all you do for ASENT.

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david.shoup@yahoo.com

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tonyaltar@gmail.com

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Children's Hospital Medical Center
blavenst@childrensnational.org

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Consulting and Neurological Services, PLLC
oneilldcruz@gmail.com

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### Schedule-at-a-glance

#### Tuesday, March 14, 2017
- 5:30 pm – 8:00 pm | ASENT Board Meeting & Dinner  
  Montrose
- 5:30 pm – 8:00 pm | Publication Committee Meeting  
  Twinbrook

#### Wednesday, March 15, 2017
- 7:30 am – 8:30 am | Coffee & Light Breakfast  
  Plaza Foyer
- 8:30 am – 9:00 am | President’s Welcome  
  Plaza II & III
- 9:00 am – 10:30 am | Pipeline Presentations Part 1  
  Plaza II & III
- 9:00 am – 5:00 pm | *All Day*- Course in Translational Research  
  Plaza I
- 10:30 am – 10:45 am | Break  
  Plaza Foyer
- 12:00 pm – 2:00 pm | Lunch with Pipeline Presenters  
  Roosevelt & Madison
- 2:00 pm – 4:00 pm | Pipeline Presentations Continue Part 2  
  Plaza II & III
- 3:30 pm – 3:45 pm | Break  
  Plaza Foyer
- 5:00 pm – 6:00 pm | Opening Reception (all)  
  Patio Foyer and Madison
- 6:00 pm – 8:30 pm | Careers in Neuroscience Dinner (all)  
  Roosevelt

#### Thursday, March 16, 2017
- 6:45 am – 7:45 am | ASENT Committee Meeting Breakfasts  
  Presidential Foyer
- 7:00 am – 8:00 am | Membership Committee Meeting  
  Monroe
- 7:00 am – 8:00 am | Communications Committee Meeting  
  Truman
- 7:30 am – 8:30 am | Coffee and Breakfast  
  Plaza Foyer
- 8:00 am – 8:30 am | President’s Announcements  
  Plaza II & III
- 8:30 am – 12:00 pm | Opening Plenary: Alzheimer Disease: Therapeutic Horizon or Mirage  
  Plaza II & III
- 10:30 am – 10:45 am | Break  
  Plaza Foyer
- 12:00 pm – 1:30 pm | Lunch with Posters and Authors  
  Roosevelt & Madison
- 1:30 pm – 3:00 pm | Plenary session – Hot Topics: Neurodegeneration: Parkinson’s Disease in 2017. What is Really New?  
  Plaza II & III
- 3:00 pm – 3:15 pm | Break  
  Plaza Foyer
- 3:15 pm – 5:15 pm | Plenary Session: Pharmacogenomics for CNS Medication Discovery, Development and Prescribing  
  Plaza II & III
- 6:00 pm – 8:30 pm | Dinner Symposium - Art, Music, Poetry and the Brain  
  Roosevelt

#### Friday, March 17, 2017
- 6:45 am – 8:15 am | ASENT Annual Meeting Committee  
  Wilson
- 7:30 am – 8:30 am | Coffee & Breakfast  
  Presidential Foyer
- 8:00 am – 5:45 pm | Registration  
  Plaza Foyer
- 8:30 am – 9:00 am | ASENT Business Meeting  
  Plaza II & III

### Friday, March 17, 2017 continued
- 9:00 am – 12:00 pm | Concurrent Symposium: Neuromodulation Update: Implantable Devices to Treat Disease/Disorders of the Central and Autonomic Nervous Systems  
  Plaza II & III
- 9:00 am – 12:00 pm | Concurrent Symposium: Pharmacologic Innovations and Drug Repurposing Repurposing Drugs for New Indications and Development  
  Plaza I
- 12:00 pm – 1:30 pm | Lunch with Posters and Authors  
  Roosevelt & Madison
- 1:30 pm – 3:00 pm | Concurrent Symposium – Hot Topics: Parkinson’s Disease in 2017. What is Really New?  
  Plaza II & III
- 1:30 pm – 3:00 pm | Concurrent Session – Hot Topics: Therapeutics in Psychiatry and Rapidly Acting Antidepressants  
  Plaza I
- 3:00 pm – 3:15 pm | Break  
  Plaza Foyer
- 3:15 pm – 5:00 pm | Closing Plenary: The Placebo Effect: Causes and Corrections for Clinical Trials  
  Plaza II & III

### Translations Research Course
**Wednesday, March 15, 2017**
- 9:00 am – 9:05 am | Overview  
  Plaza I
- 9:05 am – 9:35 am | The Healthcare Innovation Cycle and Lessons from CIMIT  
  Plaza I
- 9:35 am – 10:05 am | Defining Clinical Needs and the Value Proposition  
  Plaza I
- 10:05 am – 10:35 am | Intellectual Property  
  Plaza I
- 10:30 am – 10:45 am | Break  
  Plaza I
- 10:35 am – 11:05 am | Identifying Stakeholders in the Healthcare Ecosystem  
  Plaza I
- 11:05 am – 11:35 am | Putting Together an Action Plan from Bench to Patient  
  Plaza I
- 11:35 am – 12:00 pm | Panel Discussion-All  
  Plaza I
- 12:00 pm – 1:00 pm | Lunch Break  
  Plaza I
- 1:00 pm – 1:30 pm | “Killer” Questions and Experiments  
  Plaza I
- 1:30 pm – 2:00 pm | Clinical Trial Design  
  Plaza I
- 2:00 pm – 2:30 pm | NeuroNEX Overview and Biostatistics  
  Plaza I
- 2:30 pm – 2:45 pm | Panel Discussion-All  
  Plaza I
- 2:45 pm – 3:00 pm | Break  
  Plaza I
- 3:00 pm – 3:30 pm | Strategic Partnerships  
  Plaza I
- 3:30 pm – 4:00 pm | Regulatory Pathways  
  Plaza I
- 4:00 pm – 4:30 pm | SBIR and Other NINDS Funding Opportunities and Resources for Translational Researchers  
  Plaza I
- 4:30 pm – 4:50 pm | Panel Discussion-All  
  Plaza I
- 4:50 pm – 5:00 pm | Summary and Wrap-Up  
  Plaza I

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