FEBRUARY 8 - 12, 2021

2021 VIRTUAL COURSE
SYLLABUS

www.neurotherapeuticscourse.org
# SCHEDULE

**Monday, February 8, 2021**

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<th>Time</th>
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| 11:00 AM – 11:35 AM | **OVERVIEW: THE NEUROTHERAPEUTICS DISCOVERY AND DEVELOPMENT PROCESS**  
Michael Rogawski, MD, PhD, University of California, Davis |
| 11:35 AM – 11:45 AM | Live Discussion - Rogawski                              |
| 11:45 AM – 12:15 PM | **SESSION 1: TARGET AND PATHWAY INTERROGATION**  
James Inglese, PhD, National Center for Advancing Translational Sciences, NIH |
| 12:15 PM – 12:25 PM | Live Discussion - Inglese                                |
| 12:25 PM – 12:55 PM | **BREAK**                                                |
| 12:55 PM – 1:25 PM  | **SESSION 2: BIOLOGY BASICS FOR IDENTIFYING HITS**  
William Janzen, BS |
| 1:25 PM – 1:35 PM   | Live Discussion - Janzen                                 |
| 1:35 PM – 2:05 PM   | **SESSION 3: MOVING FROM HIT TO LEAD TO CLINICAL CANDIDATE**  
James Barrow, PhD, Johns Hopkins School of Medicine |
| 2:05 PM – 2:15 PM   | Live Discussion - Barrow                                 |
| 2:15 PM – 2:30 PM   | **BREAK**                                                |
| 2:30 PM – 3:00 PM   | **SESSION 4: ANTIBODY-BASED THERAPIES**  
Gai Ayalon, PhD, Ultragenyx Pharmaceuticals |
| 3:00 PM – 3:10 PM   | Live Discussion - Ayalon                                |
| 3:10 PM – 4:00 PM   | **MEET THE MENTORS - ZOOM BREAKOUTS**                   |
| 4:00 PM – 4:45 PM   | OFFICE HOURS WITH COURSE DIRECTORS AND FACULTY  
Please drop by if you have questions for them. |
## Schedule

**Tuesday, February 9, 2021**

<table>
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<tr>
<th>Time</th>
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| 11:00 AM – 11:05 AM | WELCOME & OVERVIEW OF THE DAY  
Barbara Slusher, MAS, PhD |
| 11:05 AM – 11:35 AM | SESSION 5: INTELLECTUAL PROPERTY  
Jeffrey W. Childers, JD, PhD, Casimir Jones S.C. |
| 11:35 AM – 11:45 AM | Live Discussion - Childers |
| 11:45 AM – 11:50 AM | INVITED GUEST SPEAKER: INTRODUCTION |
| 11:50 AM – 12:20 PM | INVITED GUEST PRESENTATION: THE STORY OF RISDIPLAM/EVRYSDI™  
Alexander Stephan, PhD, Roche Pharma |
| 12:20 PM – 12:30 PM | Live Discussion - Stephan |
| 12:30 PM – 1:00 PM | BREAK |
| 1:00 PM – 1:30 PM | SESSION 6: ADME  
Kennan Marsh, PhD, AbbVie |
| 1:30 PM – 1:40 PM | Live Discussion - Marsh |
| 1:40 PM – 2:10 PM | SESSION 7: VIGNETTE - A STORY OF "BENCH TO (ALMOST) BEDSIDE"  
Barbara Slusher, MAS, PhD, Johns Hopkins Drug Discovery |
| 2:10 PM – 2:20 PM | Live Discussion - Slusher |
| 2:20 PM – 2:35 PM | BREAK |
| 2:35 PM – 3:05 PM | SESSION 8: ALTERNATIVE APPROACHES TO LEAD GENERATION  
Sam Enna, PhD, University of Kansas Medical Center |
| 3:05 PM – 3:15 PM | Live Discussion - Enna |
| 3:15 PM – 4:15 PM | ROUND ROBINS - ZOOM BREAKOUTS |
| 4:15 PM – 5:00 PM | OFFICE HOURS WITH COURSE DIRECTORS AND FACULTY  
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<td>11:00 AM – 11:05 AM</td>
<td>WELCOME &amp; OVERVIEW OF THE DAY</td>
<td>Karl Scheidt, PhD</td>
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<td>11:05 AM – 11:25 AM</td>
<td>SESSION 9A: FORMULATION AND ROUTES OF ADMINISTRATION</td>
<td>Maureen Donovan, PhD, University of Iowa</td>
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<td>11:25 AM – 11:45 AM</td>
<td>SESSION 9B: ALTERNATIVE ROUTES OF ADMINISTRATION</td>
<td>James Cloyd III, PharmD, University of Minnesota</td>
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<td>11:45 AM – 12:00 PM</td>
<td>Live Discussion - Donovan and Cloyd</td>
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<td>12:00 PM – 12:30 PM</td>
<td>SESSION 10: TOXICOLOGY</td>
<td>Adaline Smith, PhD, DABT, Adagen Preclinical Solutions</td>
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<td>12:30 PM – 12:40 PM</td>
<td>Live Discussion - Smith</td>
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<td>12:40 PM – 1:10 PM</td>
<td>BREAK</td>
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<td>1:10 PM – 1:40 PM</td>
<td>SESSION 11: IND ENABLING STUDIES AND PREPARATION OF THE IND</td>
<td>Edward (Ted) Spack, PhD, Medared</td>
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<td>1:40 PM – 1:50 PM</td>
<td>Live Discussion - Spack</td>
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<td>1:50 PM – 2:20 PM</td>
<td>SESSION 12: RIGOR IN TRANSLATIONAL DISCOVERY AND DEVELOPMENT</td>
<td>Shai Silberberg, PhD, NINDS</td>
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<td>2:20 PM – 2:30 PM</td>
<td>Live Discussion - Silberberg</td>
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<td>BREAK</td>
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| 11:00 AM – 11:05 AM| WELCOME & OVERVIEW OF THE DAY  
Michael Rogawski, MD, PhD |
| 11:05 AM – 11:35 AM| SESSION 13: ANTISENSE OGLIONUCLEOTIDES  
Gene Liau, PhD, Stoke Therapeutics |
| 11:35 AM – 11:45 AM| Live Discussion - Liau                                                                       |
| 11:45 AM – 12:15 PM| SESSION 14: CLINICAL CONSIDERATIONS IN THE NON-CLINICAL PHASE OF DRUG DISCOVERY AND DEVELOPMENT  
James J. Vornov, MD, PhD, Johns Hopkins School of Medicine and Medpace |
| 12:15 PM – 12:25 PM| Live Discussion - Vornov                                                                      |
| 12:25 PM – 1:00 PM| BREAK                                                                                         |
| 1:00 PM – 1:30 PM | SESSION 15: FUNDING ACADEMIC DRUG DISCOVERY RESEARCH  
Charles L. Cywin, PhD, NIH/NINDS |
| 1:30 PM – 1:40 PM | Live Discussion - Cywin                                                                        |
| 1:40 PM – 2:45 PM | ROUND ROBINS - ZOOM BREAKOUTS                                                                 |
| 2:45 PM – 3:15 PM | OFFICE HOURS WITH COURSE DIRECTORS AND FACULTY  
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# Schedule

**Friday, February 12, 2021**

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| 11:00 AM – 11:05 AM | **Welcome & Overview of the Day**  
|               | Karl Scheidt, PhD                                                   |
| 11:05 AM – 1:30 PM       | **Student Presentations - Zoom Breakouts**                          |
| 1:30 PM – 2:00 PM         | **Conclusion: Where Do We Go From Here?**  
|               | **Course Evaluation and Survey**                                    |
|               | Course Directors                                                    |
| 2:00 PM               | **Adjourn**                                                          |
Thank You

The Course Directors thank the NIH for funding that makes this course possible.

NIH grant 1R25NS099170-01
Course Directors

MICHAEL A. ROGAWSKI, MD, PHD
Professor, Department of Neurology and Pharmacology, School of Medicine, University of California, Davis,
Director of the Institute for Neurotherapeutics,
Associate Director, UC Davis CounterACT Center of Excellence,
Principal Investigator, University of California Drug Discovery Consortium
rogawski@ucdavis.edu

Michael Rogawski is professor in the Department of Neurology with joint appointment in the Department of Pharmacology, and an affiliate member of the Center for Neuroscience at the University of California, Davis. Until 2006, he was senior investigator and chief of the Epilepsy Research Section at the National Institute of Neurological Disorders and Stroke. Dr. Rogawski graduated from Amherst College with a B.A. in biophysics and from Yale University with an M.D. and Ph.D. in pharmacology. After serving as a postdoctoral fellow in the Laboratory of Neurophysiology, NINDS, he completed residency training in neurology at Johns Hopkins. Dr. Rogawski was chair of the UC Davis Department of Neurology until 2012. His research encompasses cellular neurophysiological studies of ion channels (with a focus on the mechanisms of action of antiepileptic drugs); animal models of epilepsy, migraine, and nerve agent intoxication; and clinical studies on new treatments for seizures, epilepsy, migraine, traumatic brain injury, and neurodevelopmental disorders. His laboratory studies on AMPA receptors and neurosteroids have led to new treatment approaches for seizures and epilepsy, and the marketed treatment for postpartum depression brexanolone (Zulresso™). Dr. Rogawski has received the NIH Director’s Award, the Epilepsy Research Award for Outstanding Contributions to the Pharmacology of Antiepileptic Drugs from the American Society for Pharmacology and Experimental Therapeutics, the Service Award from the American Epilepsy Society, the American Academy of Neurology 2015 Neuroendocrine Research Award, and the Physician-Scientist Award of the End Epilepsy Summit, Epilepsy Foundation, Greater Los Angeles (April 2017). In October 2018, the American Association for the Advancement of Science elected Dr. Rogawski as a Fellow in the Neuroscience Section for distinguished contributions in the fields of neuroscience, neuropharmacology and neurology, particularly applied to the treatment of epilepsy. He was also elected to full membership in Sigma Xi, the scientific research honor society. In May 2019, he received the UC Davis Chancellor’s Innovator of the Year Award. In 2020, he was elected as a fellow of the National Academy of Inventors. Dr. Rogawski has served on the editorial boards of Molecular Pharmacology, Epilepsia, Epilepsy Research, Synapse, CNS Drug Reviews, Current Neuropharmacology, Pharmacology & Therapeutics, BMC Pharmacology, Cellular and Molecular Neurobiology, and Nature Scientific Reports, and was executive editor of Neuropharmacology and associate editor of Neurotherapeutics. He is a founder and was co-chief editor of Epilepsy Currents, the journal of the American Epilepsy Society. He has served on the board of directors of the American Epilepsy Society, he has been a member of advisory panels to the National Institutes of Health, and he serves in an advisory capacity as a special government employee to the Food and Drug Administration. He is past president of the American Society for Experimental Neurotherapeutics.
Course Directors

KARL SCHEIDT, PHD
Director, Northwestern Center for Molecular Innovation and Drug Discovery (CMIDD),
Professor of Pharmacology, Northwestern University,
Professor of Chemistry, Northwestern University

scheidt@northwestern.edu

Karl Scheidt is a Professor in the Department of Chemistry and Professor of Pharmacology in the Feinberg School of Medicine. His independent research program focuses on developing efficient strategies to create bioactive new molecules with translational and clinical potential. As the founding Executive Director of the NewCures Accelerator, he is responsible for developing and leading strategies to build commercial value for therapeutic discoveries within Northwestern.

Karl received a BS from the University of Notre Dame, a PhD from Indiana University and was a National Institutes of Health postdoctoral fellow at Harvard University. He is a fellow of both the Alfred P. Sloan Foundation and Royal Society of Chemistry, an American Cancer Society Research Scholar, and received multiple awards from the pharmaceutical industry. During his time at Northwestern, he has trained over 90 postdoctoral fellows and graduate students and received both a Distinguished Teaching Award and an Alumnae of Northwestern Teaching professorship, the university’s highest distinction for education. He is also the co-founder of two biotech startup companies focused on translating the promise of small molecules to clinical success.
Course Directors

BARBARA SLUSHER, MAS, PHD
Professor of Neurology (primary), Psychiatry, Neuroscience, Medicine, Pharmacology, and Oncology, Johns Hopkins School of Medicine, Director, Johns Hopkins Drug Discovery program, Adjunct Professor, Institute of Organic Chemistry and Biochemistry, Prague
bslusher@jhmi.edu

Barbara Slusher is Professor of Neurology (primary), Pharmacology and Molecular Sciences, Psychiatry, Neuroscience, Medicine and Oncology at Johns Hopkins School of Medicine and the Director of the Johns Hopkins Drug Discovery program. Dr. Slusher has published over 250 scientific articles and is the inventor on over 100 patents and applications. Before joining Johns Hopkins, Dr. Slusher spent 18 years in the pharmaceutical industry, including several years at the level of Senior Vice President of Research and Translational Development. She has extensive experience in drug discovery through early clinical development and was involved in the successful development, launch and/or post marketing support of several FDA approved medicines including Seroquel™, Aloxi™, Dacogen™, Lucendra™. In 2010, she joined Johns Hopkins to lead the largest drug discovery program on campus with a veteran team of over 25 medicinal chemists, assay developers, pharmacologists, toxicologists, and pharmacokinetics/drug metabolism experts. The team is engaged in identifying novel drug targets arising from the faculty’s research and translating them into new drug therapies for clinical development. Since joining JHU, she has co-founded four companies including Cerecor, Dracen Pharmaceuticals, Adarga, and Lorem Therapeutics and founded the first-ever International Consortium of Academic Drug Discovery with over 150 university-led translational centers and 1,500 members. Dr. Slusher has also served on multiple boards of directors, scientific advisory boards and steering committees including Janssen, Eisai, Inc., Bayer-Wilmer JSC, Bluefield Innovations, Neurofibromatosis Therapeutic Acceleration Program, Kennedy Krieger Institute, American Society for Experimental Neurotherapeutics, Longeviti Neurosolutions, and Ashvattha Therapeutics.

Dr. Slusher received her undergraduate degree from Dickinson College where she graduated valedictorian, majoring in Chemistry. She received her Ph.D. in Pharmacology and Molecular Sciences from John Hopkins School of Medicine while simultaneously earning her Master’s degree in Administrative Science from the Johns Hopkins Carey School of Business.
GAI AYALON, PHD  
Senior Director, Ultragenyx Pharmaceutical  
gai_ayalon@protonmail.com

Gai Ayalon, Ph.D., is a neuroscientist and a Senior Director at Ultragenyx Pharmaceutical, a Bay area, California company dedicated to developing therapeutics for rare and ultra-rare diseases. He is currently leading the clinical development of programs for neurodevelopmental disorders. Previously Dr. Ayalon was a scientist at Genentech in the neuroscience department. At Genentech he led three drug discovery programs focused on immunotherapeutic approaches to neurodegenerative diseases. As a basic biologist, he led a lab that investigated the mechanisms of action underlying the efficacy of therapeutic antibodies. Dr. Ayalon received his Ph.D. from the Hebrew University Medical School in Jerusalem, Israel, and conducted his postdoctoral research at Duke University Medical Center.

JAMES BARROW, PHD  
Associate Professor of Pharmacology, Johns Hopkins University,  
Primary Investigator and Director, the Drug Discovery Division at the Lieber Institute  
jbarrow@jhmi.edu

Dr. James Barrow is an Associate Professor of Pharmacology at Johns Hopkins University and a Primary Investigator and Director of the Drug Discovery Division at the Lieber Institute. Dr. Barrow has 14 years of industrial experience in drug discovery research at Merck Research Laboratories and held many leadership roles before coming to the Lieber Institute in 2010. He has published 57 peer-reviewed articles, reviews, and book chapters dealing with organic synthesis and drug discovery, most for central nervous system disorders, as well as 33 issued patents. He was part of development teams at Merck that brought new chemical entities through toxicology studies and into Phase 1 and 2 clinical trials. At the Lieber Institute, he has continued to lead drug discovery teams looking for treatments of psychiatric disorders. Dr. Barrow received a B.S. in chemistry from the University of North Carolina, Chapel Hill, graduating with highest honors and highest distinction. He received his Ph.D. from Harvard University, working in the laboratory of Professor David A. Evans.
JEFFREY W. CHILDERS, JD, PHD
Shareholder, Casimir Jones, S.C.
jwchilders@casimirjones.com

Jeff Childers is a shareholder with Casimir Jones, S.C., and has more than fifteen years of experience preparing and prosecuting U.S. and foreign patent applications in pharmaceuticals, specialty chemicals, molecular diagnostics, biosensors, textiles, material sciences, medical devices, and related areas. He also provides clients with due diligence analyses, including patentability, freedom-to-operate, and infringement/non-infringement opinions; provides guidance and counseling on the intellectual property aspects of licensing and business transactions; and provides strategic advice and management of patent portfolios.

Dr. Childers also has significant experience with university technology transfer, including serving as in-house patent counsel with Johns Hopkins Technology Transfer and completing an externship with the University of North Carolina Office of Technology Development. He has a Ph.D. in chemistry from Duke University and graduated with honors from the University of North Carolina School of Law.

JAMES CLOYD III, PHARMD
Morse Alumni Distinguished Teaching Professor,
Professor of Neurology and Pharmacy,
Lawrence C. Weaver Endowed Chair in Orphan Drug Development,
Director of the Center for Orphan Drug Research,
University of Minnesota College of Pharmacy
cloyd001@umn.edu

James Cloyd, PharmD, is a University of Minnesota Morse Alumni Distinguished Teaching Professor, a Professor of Neurology and Pharmacy, the Lawrence C. Weaver Endowed Chair in Orphan Drug Development, and Director of the Center for Orphan Drug Research at the University of Minnesota College of Pharmacy. Dr. Cloyd co-authored the orphan drug application for diazepam rectal gel (Diastat) and was a leader in the development of the product. His research interests include: orphan drugs, clinical neuropharmacology, antiepileptic drugs, and rare pediatric neurological disorders. He serves on the FDA clinical pharmacology advisory committee and previously was a member of the American Epilepsy Society Board of Directors and the editorial board of Epilepsia. He is a recipient of the J. Kiffin Penry Award for Excellence in Epilepsy Care from the American Epilepsy Society, the Rho Chi Lecture Award, the Summer J. Yaffe Lifetime Achievement Award in Pediatric Pharmacology and Therapeutics, and the American Pharmacists Association Tyler Prize for Stimulation of Research.
Charles L. Cywin, PhD
Program Director for the Blueprint Neurotherapeutics Network (BPN), National Institute of Neurological Disorders and Stroke (NINDS)
cywincl@mail.nih.gov

Dr. Charles L. Cywin joined the National Institute of Neurological Disorders and Stroke (NINDS) in 2011 in the Office of Translational Research and currently serves as the Program Director for the Blueprint Neurotherapeutics Network (BPN), which has produced 6 successful INDs advancing to Phase I trials with 3 projects already now in Phase II testing. In 2018 he additionally took on the HEAL Initiative: Non-addictive Analgesic Therapeutics Development [Small Molecules and Biologics] to Treat Pain Program. He is responsible for the providing project leadership and coordination for a portfolio of neuroscience and pain drug discovery projects ranging from early discovery to Phase I trials. He is the Contract Officer Representative for several large NIH R&D contracts. Prior to joining NIH he spent 17 years at Boehringer Ingelheim Pharmaceuticals where he was Director, Medicinal Chemistry and was responsible for Hit to Lead and Lead Optimization programs focused on successful clinical candidate selection in a number of therapeutic areas. He created the lead discovery team, the parallel synthesis group, and led the efforts to identify and implement new technologies, infrastructure, and outsourcing in medicinal chemistry.

Dr. Cywin received his Bachelor’s degree in chemistry from Providence College, performed his doctoral training at Syracuse University focused on natural product synthesis where he was a University Fellow, and completed his postdoctoral fellowship with Professor E.J. Corey at Harvard focusing on asymmetric catalysis.

Maureen Donovan, PhD
Professor, Department of Pharmaceutical Sciences and Experimental Therapeutics, The University of Iowa College of Pharmacy
maureen-donovan@uiowa.edu

Maureen Donovan is a Professor of Pharmacy at the University of Iowa. She leads a research group investigating drug absorption mechanisms, in particular, the pathways available in the nasal mucosa leading to distribution to the systemic circulation, lymphatics, and into the CNS via the olfactory and trigeminal nerves and associated supporting cells. Dr. Donovan has extensive experience in and teaches formulation development strategies to accomplish drug delivery at a wide variety of administration sites. Dr. Donovan has directed over 50 M.S. and Ph.D. students and visiting scholars, many of whom are employed as formulation development scientists across the pharmaceutical and biotechnology industries. She chaired the NIH Emerging Technologies in Neuropharmacology/Neuroscience SBIR study section from 2009-2011 and again from 2017-2019. She currently serves as a member of the FDA Pharmaceutical Sciences and Clinical Pharmacology Advisory Committee. Professor Donovan received her B.S. in Pharmacy from the University of Minnesota (1983) and a Ph.D. in Pharmaceutics from the University of Michigan (1989).
SAM J. ENNA, PHD
Professor Emeritus of Pharmacology and of Physiology,
The University of Kansas Medical Center
senna@kumc.edu
Dr. S. J. Enna is Professor Emeritus of Physiology and of Pharmacology at the University of Kansas Medical Center. Previous appointments include Chair of Pharmacology and Associate Dean for Research at the University of Kansas, Professor of Pharmacology and Neurobiology at the University of Texas Medical School at Houston, Lecturer in Neuroscience at Johns Hopkins University School of Medicine, and Adjunct Professor of Pharmacology at Tulane University School of Medicine. He has served as a consultant with ICI-USA, Inc., Merck, Sharp and Dohme Research Laboratories, Bristol-Myers Corporation, Panlabs, and as a member of the Scientific Advisory Council of Abbott Laboratories. He spent six years as Senior Vice President, Scientific Director, and Executive Vice President of Nova Pharmaceutical Corporation. Dr. Enna made significant contributions in defining the pharmacological and biochemical properties of neurotransmitter receptors. He has received numerous awards for his research, including the John Jacob Abel, Torald Sollmann, Otto Krayer, and Daniel H. Efron Awards, a PhARMA Foundation Excellence Award, and the Paolelli Medal. He has been appointed an Honorary Fellow of the British Pharmacological Society, as a Fellow of the American Society for Pharmacology and Experimental Therapeutics, and as an Honorary Member of the Chinese Pharmacological Society. Elective offices include the presidency of the American Society for Pharmacology and Experimental Therapeutics, and as Secretary General and then President of the International Union of Basic and Clinical Pharmacology (IUPHAR). Dr. Enna served as editor of The Journal of Pharmacology and Experimental Therapeutics and is currently co-editor of Current Protocols in Pharmacology, Editor-in-Chief of Biochemical Pharmacology, Executive Editor-in-Chief of Pharmacology and Therapeutics and Series Editor of Advances in Pharmacology.

JAMES INGLESE, PHD
Principal Investigator, National Center for Advancing Translational Sciences (NCATS),
Adjunct Investigator, National Human Genome Research Institute (NHGRI),
National Institutes of Health (NIH)
jinglese@mail.nih.gov
James Inglese, Ph.D. is a Principal Investigator within the National Center for Advancing Translational Sciences (NCATS) and Adjunct Investigator of the National Human Genome Research Institute (NHGRI). His laboratory is focused on the development of assay and screening technology targeting the discovery of chemical probes and therapeutic leads for the study and treatment of rare and neglected disease. The NCATS post-doctoral training program he has established is recognized by disease foundations as an effective collaborative bridge to NCATS expertise and resources enabling a champion-driven effort in early stage translation. Prior to the formation of NCATS, he co-founded the NIH Chemical Genomics Center (NCGC) acting as its Deputy Director. Dr. Inglese received his Ph.D. in Organic Chemistry from the Pennsylvania State University and completed postdoctoral training in the laboratory of 2012 Nobel Laureate Prof. Robert J. Lefkowitz at Duke University Medical Center. Before coming to the NIH, Dr. Inglese led research teams at the combinatorial chemistry Princeton-based biotech Pharmacopeia and Merck Research Laboratories. Over the past two decades, Dr. Inglese has contributed to over 180 publications and patents; his efforts on the early drug discovery process have resulted in novel assay formats, reporter systems, and high throughput screening paradigms. Dr. Inglese serves on the scientific advisory boards of several NIH-funded chemistry and screening centers, disease foundations, and international chemical biology consortia.
WILLIAM JANZEN, BS
bjanzen@bellsouth.net

Bill Janzen recently retired from a long career that spanned over thirty years in drug discovery and now engages in limited consulting activities. Most recently he was the Vice President of Lead Discovery and Research Operations at Ribometrix, a platform therapeutics company discovering small molecule drugs that target functional 3D RNA structures to treat human diseases. He has been a leader in the field of lead generation in academia, start-up companies, and large pharmaceutical ventures in both executive and operational positions including Executive Director of Lead Generation at Epizyme, Director of Assay Development and Compound Profiling at the University of North Carolina, President and Chief Operating Officer of Amphora Discovery, and Director of Lead Generation Technologies at Eli Lilly and Company.

Bill was one of the founders of the Society for Biomolecular Screening (SBS) and served as president from 1998 – 2000. He served on the board of directors of the SBS and Society for Laboratory Automation and Screening. He assembled the definitive work on high throughput screening, *High Throughput Screening Methods and Protocols* and has subsequently edited second and third editions of the book. Bill has worked on over 500 drug discover projects in his career including numerous projects that have entered clinical trials.

Bill served as a scientific advisor to multiple government agencies including the National Toxicology Program where he chaired the Scientific Advisory Committee on Alternative Toxicological Methods. He has also participated in and chaired multiple review committees for government grants and contracts and corporate scientific advisory boards.

GENE LIAU, PHD
EVP, Head of Research & Preclinical Development, Stoke Therapeutics
gliau@stoketherapeutics.com

Gene Liau is executive vice president, head of research and preclinical development at Stoke Therapeutics. Gene was most recently senior vice president and head of gene therapy R&D at Precision BioSciences. Prior to Precision BioSciences, Gene spent five years at Pfizer where, as executive director, he led external R&D efforts for rare disease and hematology programs and spearheaded the company’s gene therapy initiative. Gene joined Pfizer from Shire, where he helped the company to build an innovative rare disease portfolio. Gene also spent 12 years at Novartis, initially building a research unit focused on using gene therapy for cardiovascular and metabolic applications and eventually leading the cardiovascular/metabolic division at the Novartis Institutes for BioMedical Research in Cambridge, where he was responsible for projects from target discovery to early clinical proof of concept. Prior to entering industry, Gene was a Professor at George Washington University Medical Center and also a senior scientist at the Jerome H. Holland Laboratory of the American Red Cross. Gene received his Ph.D. in biochemistry from Vanderbilt University and completed his postdoctoral fellowship at the National Cancer Institute.
KENNAN MARSH, PHD  
Distinguished Research Fellow, AbbVie  
kennan.marsh@abbvie.com

Kennan Marsh joined Abbott in 1982, with a B.A. in chemistry from Hollins and a PhD in Pharmaceutical Chemistry from the University of Kansas. At Abbott/AbbVie she formed and led the Nonclinical Pharmacokinetics group, supporting the evaluation of compounds from Discovery project teams, assisting in the integration of physical chemical properties, metabolism and pharmacokinetics in the discovery and development of multiple small molecule compounds which entered clinical trials (e.g., ritonavir, kaletra, venetoclax, pibrentasvir, gelcaprevir). Kennan is currently a Distinguished Research Fellow, assisting the Neglected Tropical Diseases team and our partners (MMV, DNDi, AWOL) in the development of compounds for diseases such as malaria, TB, and a variety of filarial diseases.

ADALINE SMITH, PHD, DABT  
Adagen Preclinical Solutions, LLC  
smithacs35@gmail.com

Adaline Smith is a Board Certified Toxicologist who has been involved in drug development efforts in both the public and private sector for more than 25 years. She has extensive experience in the development of drugs across a broad range of therapeutic areas, including neurology, oncology, cardiovascular, hematology, and gastrointestinal indications. She has been responsible for leading, providing technical oversight, and strategic guidance for toxicology, pharmacology, and DMPK functional areas at every phase of drug development, from drug discovery efforts into regulated studies. As part of the drug development effort, she provided nonclinical guidance and strategic recommendations to progress and support global program development. Dr. Smith has been responsible for the nonclinical studies which led to more than 20 successful regulatory filings to support clinical trials and marketing applications.
EDWARD (TED) SPACK, PHD
Principal, Vector Biosciences Preclinical Consulting,
VP Research and Corporate Development, AfaSci Inc.
egspack@comcast.net

Dr. Edward (Ted) Spack has over 30 years of biotech translational experience, including preclinical development of drug candidates for multiple sclerosis, nosocomial infection, and biodefense. He has worked on biologic and small drug development projects in Alzheimer’s disease, multiple sclerosis, cystic fibrosis, melanoma, and pseudomonas infection. In addition to his research and development work, he has experience in business development, including in-and out-licensing of novel technologies, and co-founding a start-up biotech company focused on neuroinflammation. He has a longstanding interest in translation of academic discoveries into the clinic and models for bridging the preclinical “Valley of Death.” At SRI International, Dr. Spack directed the PharmaSTART program (a consortium of SRI, Stanford, UC Berkeley, UC San Diego, and UC San Francisco), drafting preclinical development blueprints that led to several major grants and new biotech companies. As Managing Director of an innovative partnership between the National Multiple Sclerosis Society and EMD Serono he supported early-stage MS drug discovery and development projects in academic labs and biotech companies. He has consulted with the NIH translational core services committee and several NIH institutes on preclinical development and serves on several translational study sections, including the NIA Alzheimer’s Disease Drug Development review panel (chair), the NIH Small Business Review on Drug Discovery for Aging, Neuropsychiatric and Neurological Disorders, and the Alzheimer’s Drug Discovery Foundation. Through the California Life Sciences Institute (CLSI) FAST program he mentors San Francisco Bay area and global academic and industry teams in biotech company formation and pitch decks. He currently consults for the company he co-founded and for several other biotechs focused on neurodegenerative disease in addition to serving as V.P. of Research and Corporate Development for a company developing treatments for neuropathic pain. Dr. Spack received his doctoral degree from The Johns Hopkins University and his postdoctoral fellowship in cellular immunology at Stanford University.

JAMES VORNOV, MD, PHD
Vice President, Medical Affairs, Medpace,
Adjunct Assistant Professor, Johns Hopkins Drug Discovery
jvornov1@jhmi.edu

Dr. Vornov is an internationally known clinician-scientist with broad knowledge of both neuroscience and drug development. He has worked in multiple CNS therapeutic areas having directed programs in Depression, Suicidal Ideation, Parkinson’s Disease treatment and diagnosis, stroke, neuropathic pain, diabetic and chemotherapy-induced peripheral neuropathies, anesthesia and brain tumors, with particular expertise in the rapid transition of compounds from the laboratory to clinical proof of concept through the use of Critical Path technologies such as biomarkers, PK/PD modeling, adaptive design, and clinical trial simulation. Dr. Vornov has successfully brought multiple compounds into man to proof of concept and successful NDA submission, providing broad clinical trial design expertise, clinical pharmacology experience, operational excellence and global regulatory strategy development across a broad range of CNS diagnostics and therapeutics. Prior to joining Medpace, Dr. Vornov was Chief Medical Officer and SVP of Clinical Development at a private, development stage biopharmaceutical company. He provides Drug Development expertise to the Johns Hopkins Drug Discovery Program at the Johns Hopkins Medical School. Dr. Vornov received his B.A. in biology from Columbia University and his M.D. and Ph.D. from Emory University School of Medicine. He trained in Neurology at the Johns Hopkins Medical School where he served on the faculty for 10 years prior to transitioning to industry.
Dr. Stephan received his undergraduate degree in Biology from the University of Hamburg, Germany, and his PhD in Biochemistry and Neuroscience from the University of Zurich, Switzerland. He then did his post-doctoral training with Ben A. Barres at Stanford University School of Medicine, USA, where he focused on neuron-glia interactions and complement-mediated CNS plasticity in neural development, aging and injury. Dr. Stephan joined industry first as a senior lab scientist then later as a biology project leader in GlaxoSmithKline’s (GSK) Neural Pathways DPU in Singapore, where he was engaged in advancing disease modifying small molecule drugs for neurodegenerative diseases, in particular for Amyotrophic Lateral Sclerosis (ALS). He then moved to a project leader role at Merck Research Laboratories of Merck & Co. where he led global oncology in vitro discovery pharmacology projects as well as collaborative target validation efforts with academics and other external partners in neuroscience and oncology drug discovery. Dr. Stephan joined Roche’s Rare Disease discovery group in 2017 as non-clinical pharmacology lead for risdiplam/Evrysdi™. As Research Project Leader, he has also been responsible for leading preclinical drug discovery teams to discover novel exploratory drugs and to move these from discovery stages until handover for clinical development. Since October 2020, Dr. Stephan is now leading within Roche’s Neuroscience and Rare Disease DTA a cross-functional drug development team responsible for early clinical development of a novel disease modifying drug candidate for a neurodegenerative disease.
MENTORING

Mentor Roster & Schedule:

<table>
<thead>
<tr>
<th>Mentor Team</th>
<th>Mentors</th>
<th>Students</th>
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<tbody>
<tr>
<td>Room A</td>
<td>Gai Ayalon</td>
<td>Constanza Cortes</td>
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<td></td>
<td>Konnan Marsh</td>
<td>Erik Garcia</td>
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<td>Anthony Patrizzi</td>
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<td>Shuli Xia</td>
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<td>Room B</td>
<td>James Vornov</td>
<td>Tameka Clemons</td>
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<td></td>
<td>Barbara Slusher</td>
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<td>Benedict Kolber</td>
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<td>Yohannes Woldeamanual</td>
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<td>Room C</td>
<td>Michael Rogawski</td>
<td>Erin Anderson</td>
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<td>Maureen Donovan</td>
<td>Aji Alex Reynold</td>
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<td>Amber Kelley</td>
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<td>Alexander Vasin</td>
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<td>Room D</td>
<td>James Barrow</td>
<td>Run-Duo Gao</td>
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<td></td>
<td>James Inglese</td>
<td>Emily Thompson</td>
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<td>Elizabeth Johnson</td>
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<td>Room E</td>
<td>Karl Scheidt</td>
<td>Ashish Dhar</td>
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<td>Gonzalo Otazu</td>
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<td>Joshua Gross</td>
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<td>Long-Jun Wu</td>
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<td>Room F</td>
<td>Bill Janzen</td>
<td>Marco Carpenter</td>
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<td>Sam Enna</td>
<td>Filomena Pirozzi</td>
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<td>Rory Morgan</td>
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<td>Room G</td>
<td>Ted Spack</td>
<td>Carlos Barrero</td>
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<td>Gene Liau</td>
<td>Brett Morrison</td>
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<td>Lauren Jantzue</td>
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<td>Gihyun Yoon</td>
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<td>Room H</td>
<td>James Cloyd</td>
<td>Avery Frenzen</td>
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<td>Adaline Smith</td>
<td>Matthew Rhodes</td>
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<td>Nora Gray</td>
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<td>Marisa Zallocchi</td>
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</tbody>
</table>
PARTICIPANTS

2021 Course Participants

ERIN LEIGH ANDERSON
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Project: Sex differences in the mesolimbic reward system microglial response in nicotine use disorder

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Project: Exercise your Brain: Skeletal Muscle Circulating Factors as Novel Exercise-Mimetics in Alzheimer’s Disease

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Access To The Course

COURSE ZOOM:
https://us02web.zoom.us/meeting/register/tZwoduCuqToiEtQ0YqS0VuSds7YKcTqlMyfnZ

- You must register using this link to receive your unique access link. Please do so prior to the course.

Questions?

Program questions: Caroline Foote caroline@asent.org

Technical/access questions: asent@target-conferences.com

Course website: www.neurotherapeuticscourse.org

Feedback

Participants are asked to provide feedback about the course during the last session on Friday. It is very important that you complete the survey at the conclusion of the course.
Attend the ASENT 2021 Annual Meeting

Course Alumni and Faculty are encouraged to attend the ASENT2021 Annual Meeting, which will take place virtually February 22-25, 2021.

Attendees will not only have the opportunity to learn outstanding scientific content but participants will enjoy a robust networking platform, posters, and pipeline sessions, as well.

The ASENT2021 Annual Meeting is the only unique forum in American Neurology which brings together divergent areas of effort from industry, academia, advocacy, and government on one platform with open dialogue. We are pleased to welcome our faculty who include executive-level biohealth industry leaders, chairs of neurology and clinician scientists from leading academic institutions from around the world, chief scientific officers from advocacy organizations leading the way in funding neurotherapeutics, and leaders from NIH, FDA, NINDS and many other agencies.