Training in
Neurotherapeutics Discovery and Development
for academic scientists

MARCH 28 - 31, 2022

2022 COURSE SYLLABUS

www.neurotherapeuticscourse.org

GENERAL INFORMATION

- **Course website** - www.neurotherapeuticscourse. org
  - Wifi information
  - Mentor teams
  - Round Robin and Thursday presentation schedules
  - COVID policy
  - Maps

- **Wifi** - please set up an account to access the conference center wifi. You can find information on the course website.

- **COVID policy** - please follow the COVID protocols emailed to you prior to the course. These are also posted on the course website.

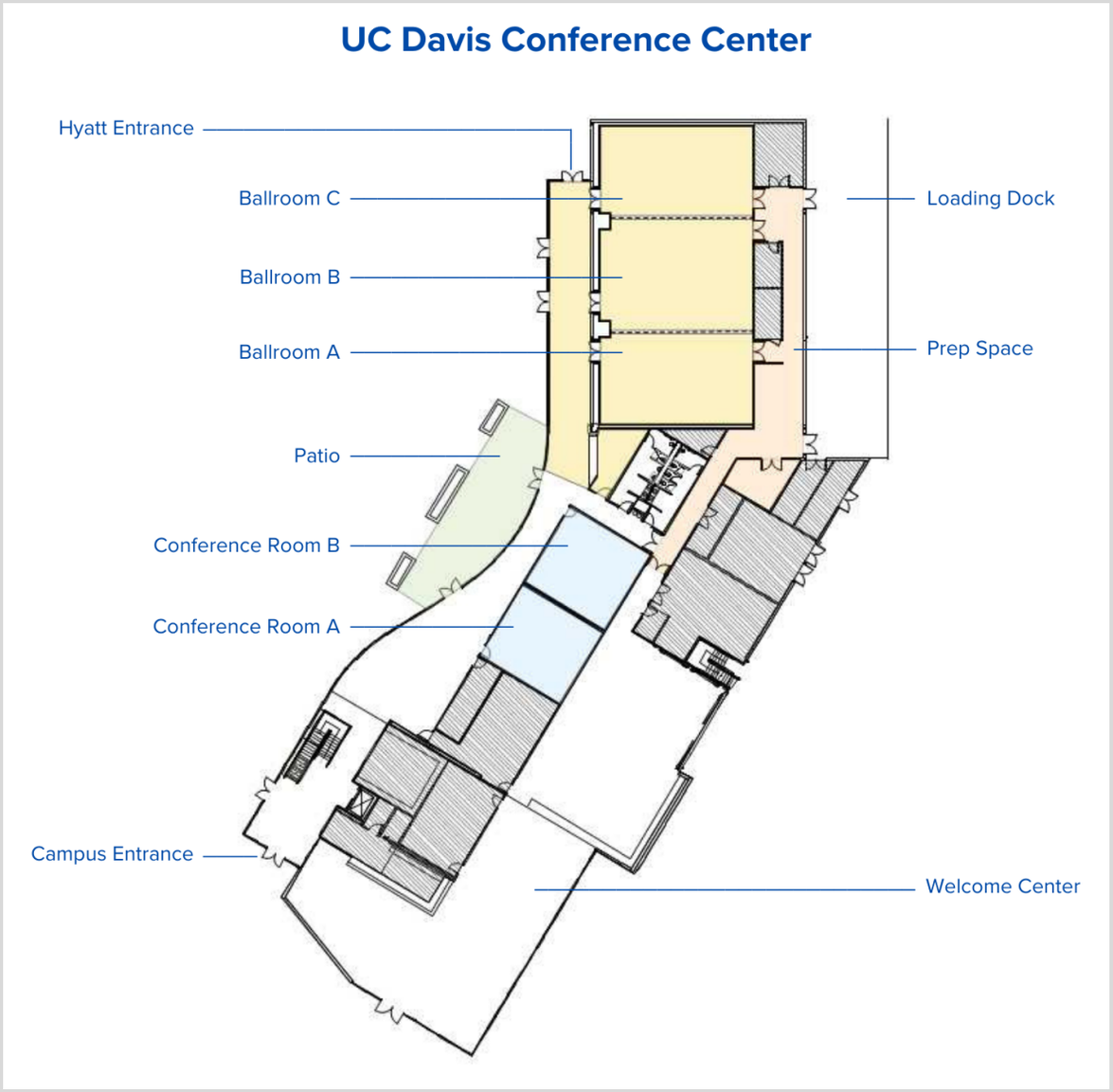
- **On site contact** - Angie Rubin - arubin12@jhmi.edu

Course website

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

NIH grant 1R25NS099170-01

MAP



- Lectures held in Ballroom B
- Breakouts held in Conference Rooms A & B and Ballrooms A & C

SCHEDULE

All times Pacific

Monday, March 28, 2022

7:15 AM - 8:00 AM	**CHECK-IN AND CONTINENTAL BREAKFAST**	
8:00 AM – 8:30 AM	**Overview: The Neurotherapeutics Discovery and Development Process** Michael Rogawski, MD, PhD, University of California, Davis	Ballroom B
8:30 AM - 9:20 AM	**Session 1: Target and Pathway Interrogation** James Inglese, PhD, National Center for Advancing Translational Sciences, NIH	Ballroom B
9:20 AM - 10:10 AM	**Session 2: Biology Basics for Identifying Hits** William Janzen, BS	Ballroom B
10:10 AM - 10:30 AM	**BREAK**	
10:30 AM - 11:20 AM	**Session 3: Moving from Hit to Lead to Clinical Candidate** James Barrow, PhD, Johns Hopkins School of Medicine	Ballroom B
11:20 AM - 12:10 PM	**Session 4: Antibody-Based Therapies** Gai Ayalon, PhD, Neumora Therapeutics	Ballroom B
12:10 PM - 1:00 PM	**LUNCH** Meet with Mentors	Ballrooms A&C Conference rooms A&B
1:00 PM - 1:50 PM	**Session 5: Intellectual Property** Jeffrey W. Childers, JD, PhD, Casimir Jones	Ballroom B
1:50 PM - 2:40 PM	**Session 6: ADME** Kennan Marsh, PhD, AbbVie	Ballroom B
2:40 PM - 3:00 PM	**BREAK**	
3:00 PM - 3:15 PM	**Introduction to Round Robins** Course Directors	Ballroom B
3:15 PM- 4:15 PM	**Engagement Exercise: Round Robin Day 1**	Ballrooms A&C Conference rooms A&B
4:45 PM - 6:00 PM	**BREAK**	
6:00 PM - 8:00 PM	**DINNER**	

SCHEDULE

Tuesday, March 29, 2022

7:15 AM - 8:00 AM	**CONTINENTAL BREAKFAST**	
8:00 AM – 8:50 AM	**Session 7: Formulation and Routes of Administration** Maureen Donovan, PhD, University of Iowa	Ballroom B
8:50 AM – 9:40 AM	**Session 8: Alternative Routes of Administration** James Cloyd III, PharmD, University of Minnesota	Ballroom B
9:40 AM - 10:00 AM	**BREAK**	
10:00 AM - 10:50 AM	**Session 9: Alternative Approaches to Lead Generation** S.J. Enna, PhD, University of Kansas	Ballroom B
10:50 AM - 11:40 AM	**Session 10: Toxicology** Adaline Smith, PhD, DABT, Adagen Preclinical Solutions	Ballroom B
11:40 AM - 1:50 PM	**LUNCH** Responsible Conduct of Research Exercise	Ballrooms A&C Conference rooms A&B
1:50 PM - 2:40 PM	**Session 11: IND Enabling Studies and Preparation of the IND** Ted Spack, PhD, Vector Biosciences	Ballroom B
2:40 PM – 3:10 PM	**Session 12: Academic Drug Discovery Vignette - A story of "Bench to (Almost) Bedside"** Barbara Slusher, PhD, MAS, Johns Hopkins	Ballroom B
3:10 PM - 3:25 PM	**Introduction to Round Robins** Mentor Teams	Ballroom B
3:25 PM - 3:45 PM	**BREAK**	
3:45 PM- 4:45 PM	**Engagement Exercise: Round Robin Day 2**	Ballrooms A&C Conference rooms A&B
4:45 PM - 6:00 PM	**BREAK**	
6:00 PM - 8:00 PM	**DINNER**	

SCHEDULE

Wednesday, March 30, 2022

7:15 AM - 8:00 AM	**CONTINENTAL BREAKFAST**	
8:00 AM – 8:50 AM	**Session 13: Antisense Oligonucleotides** Gene Liau, PhD, Stoke Therapeutics	Ballroom B
8:50 AM – 9:40 AM	**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	Ballroom B
9:40 AM - 10:00 AM	**BREAK**	
10:00 AM - 10:50 AM	**Session 15: Rigor in Translational Drug Discovery and Development – VIRTUAL** Shai Silberberg, PhD, NINDS	Ballroom B
10:50 AM - 11:40 AM	**Session 16: Funding Academic Drug Discovery Research – VIRTUAL** Charles L. Cywin, PhD, NINDS	Ballroom B
11:40 AM - 1:00 PM	**LUNCH**	
1:00 PM - 2:30 PM	**Engagement Exercise: Round Robin Day 3**	Ballrooms A&C Conference rooms A&B
2:30 PM - 4:30 PM	**Presentation Work - Time for students to work on presentations;** mentors available for questions	Ballrooms A&C Conference rooms A&B
4:30 PM - 6:00 PM	**BREAK**	
6:00 PM - 8:00 PM	**DINNER**	Mondavi Center

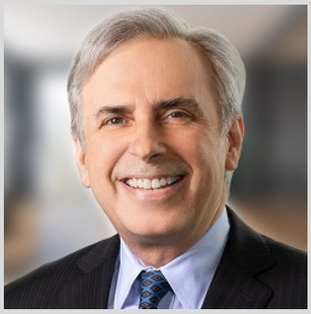
SCHEDULE

Thursday, March 31, 2022

7:15 AM – 8:00 AM	**CONTINENTAL BREAKFAST**	
8:00 AM – 8:15 AM	**STUDENT PRESENTATIONS – INTRO** Course Directors	Ballroom B
8:15 AM – 11:30 AM	**ENGAGEMENT EXERCISE: STUDENT PRESENTATIONS**	Ballrooms A&C Conference rooms A&B
11:30 AM - NOON	**CONCLUSION: WHERE DO WE GO FROM HERE?** Course Directors • Course Evaluations • Boxed lunches available	Ballroom B
NOON	**COURSE ADJOURNS**	

FACULTY

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.

FACULTY

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.

FACULTY

Course Directors

BARBARA SLUSHER, PHD, MAS
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™, Lucedra™.

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.

FACULTY

GAI AYALON, PHD
VP, Head of Portfolio and Program Management, Neumora Therapeutics

gai_ayalon@protonmail.com

Gai Ayalon, Ph.D., is a neuroscientist, Vice President, Head of the Portfolio and Program Management group at Neumora Therapeutics. He oversees the portfolio strategy across preclinical and clinical stage programs and is leading clinical stage teams developing treatments for neurological disorders. Previously he worked at Ultragenyx Pharmaceutical, where he led clinical development teams and programs for pediatric neurodevelopmental disorders. Prior to Ultragenyx, 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
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 a Professor of Pharmacology at Johns Hopkins University and a Primary Investigator and Associate 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 over 60 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.

FACULTY

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), was a leader in the development of the product, and continues to study novel formulations of CNS drugs. 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.

FACULTY

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.

FACULTY

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).

FACULTY

S.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 Paoletti 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*.

FACULTY

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.edu

Dr. 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 post-doctoral training in the laboratory of Prof. Robert J. Lefkowitz at Duke University Medical Center where he cloned and studied the regulatory G protein-coupled receptor kinases (GRKs). 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 200 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 has served on the scientific advisory boards of NIH-funded chemistry and screening centers, disease foundations, and international chemical biology consortia. Further information can be found at https://en.wikipedia.org/wiki/James_Inglese

FACULTY

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.

FACULTY

GENE LIAU, PHD
Chief Scientific Officer, Stoke Therapeutics

gliau@stoketx.com

Gene Liau is Chief Scientific Officer at Stoke Therapeutics. Gene was most recently Senior Vice President and head of gene therapy R&D at Precision BioSciences. Prior to Precision BioSciences, he spent four 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 all programs in this area at the Novartis Institutes for BioMedical Research, Cambridge from target discovery to early clinical proof of concept. Prior to entering industry, Gene was a Professor at George Washington University Medical Center and 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

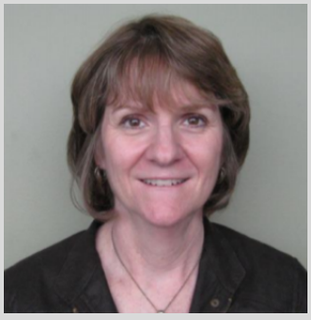
kennan.marsh@abbvie.com

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.

FACULTY

SHAI D. SILBERBERG, PHD
Director for Research Quality
National Institute of Neurological Disorders and Stroke (NINDS)
National Institutes of Health (NIH)
silberbs@ninds.nih.gov

Dr. Shai D. Silberberg is the Director for Research Quality at the NIH National Institute of Neurological Disorders and Stroke (NINDS) leading the Institute efforts to increase the excellence of science and the completeness of research reporting. Dr. Silberberg obtained a Ph.D. in Neurophysiology from the Hebrew University in Jerusalem. Prior to joining NINDS, Dr. Silberberg was an Associate Professor at Ben-Gurion University of the Negev in Israel, investigating the biophysical functions and physiological roles of ion channels.



ADALINE C. 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.

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EDWARD (TED) SPACK, PHD
Principal, Vector Biosciences Preclinical Consulting

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.

FACULTY

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.

PARTICIPANTS

2022 Course Participants

NICHOLAS SCOTT AKINS, PHD
Postdoctoral Research Associate, Chemistry
Emory University

nakins@emory.edu

Project: The Pharmacokinetic Optimization of Novel MDMA-like Compounds through a Comprehensive Structure Activity Relationship Study

ANDREW ARCH BOLINGER, PHD
Post Doctorate, Pharmacology and Toxicology
University of Texas Medical Branch

aaboling@utmb.edu

Project: 5-HT2AR Positive Allosteric Modulators as Neurotherapeutics

LAUREN DONOVAN, PHD
Postdoctoral Fellow, Anesthesiology, Perioperative and Pain Medicine
Stanford University

ljd14@stanford.edu

Project: Senolytics for the Treatment of Neuropathic Pain

KRYSTAL HERLINE-KILLIAN, PHD
Postdoctoral Research Scientist,
Taub Institute for Research on Alzheimer's Disease and the Aging Brain
Columbia University

kmh2232@cumc.columbia.edu

Project: Inhibition of a transcription factor heterodimer upregulated in A

ILYAS BERHANE, PHD
Postdoctoral Fellow, Brain Science Institute - Drug Discovery
Johns Hopkins Medicine

iberhan1@jhmi.edu

Project: Discovery of Mas-related G protein-coupled receptors-X1 (MRGPRX1) modulators for the treatment of neuropathic pain

ROBERT DAMOISEAUX, PHD
Professor, Molecular and Medical Pharmacology
UCLA

rdamoiseaux@mednet.ucla.edu

Project: Brain-permeable antivirals against Enterovirus D-68

MARCO I. GONZÁLEZ, PHD
Associate Professor, Neurology
University of California, Davis

migonzalezc@ucdavis.edu

Project: The role of calpain-dependent proteolysis in epileptogenesis

MADISON JAMES, MS
PhD Candidate, Pathobiology Program
Johns Hopkins School of Medicine

mjames34@jhmi.edu

Project: iPSC Co-culture System to Evaluate ALS Therapeutics Targeting the NMJ

PARTICIPANTS

2022 Course Participants

JING JIN, MD, PHD
Instructor, Psychiatry and Behavioral Neuroscience
Johns Hopkins University School of Medicine

jjin11@jhmi.edu

Project: Small molecules targeting mutant huntingtin posttranslational modification

ELIZABETH JOHNSON
Graduate Student,
Northwestern University

elizabethjohnson2021@u.northwestern.edu

Project: Development of Dexras1 nitrosylation inhibitors to prevent iron-induced damage in Multiple Sclerosis

BRITTANY KARAS, PHD
Post Doctoral Fellow, Neuroscience and Cell Biology
Rutgers Robert Wood Johnson Medical School

bkaras@rwjms.rutgers.edu

Project: Drug screening novel therapies in zebrafish for rare forms of congenital muscular dystrophy, dystroglycanopathies.

VIRGINIA KIMONIS, MD
Principal Investigator, Pediatrics
University of California, Irvine

vkimonis@hs.uci.edu

Project: Translational Studies in Rare Genetic Disorders

AMOL KULKARNI, PHD
Associate Professor, Pharmaceutical Sciences
Howard University

amol.kulkarni@howard.edu

Project: Novel NLRP3 inflammasome inhibitors for treatment of HIV-associated neurocognitive disorders

VIVEK KUMAR, PHD
Associate Professor, Mammalian Genetics
The Jackson Laboratory

vivek.kumar@jax.org

Project: Psychiatric drug discovery using advanced behavioral screens in mice

FERNANDA LAEZZA, MD, PHD
Professor, Department of Pharmacology and Toxicology
University of Texas Medical Branch

felaezza@utmb.edu

Project: FGF13 ligands for the treatment of substance use disorder

JENNA LIBERA
Graduate Student, Pharmacology & Experimental Therapeutics,
Boston University

jlibera1@bu.edu

Project: METTL3 inhibition as a therapeutic mechanism to alleviate tau burden and cognitive decline in Alzheimer's Disease

PARTICIPANTS

2022 Course Participants

SAMUEL MACKENZIE, MD, PHD
Senior Lecturer, Neurology
University of Rochester
samuel_mackenzie@urmc.rochester.edu

Project: Targeting somatic expansion in myotonic dystrophy type 1

ROGER NARAYAN, PHD
Professor, Biomedical Engineering
North Carolina State University
rjnaraya@ncsu.edu

Project: Transdermal Delivery of an Ampakine for Maintenance of Respirations

RUDRA PANGENI, PHD
Postdoctoral Fellow, Pharmaceutics
Virginia Commonwealth University
pangenir@vcu.edu

Project: Berberine/β-cyclodextrin inclusion complex loaded self-emulsifying drug delivery system for the treatment of chemotherapy-induced neuropathic pain

FRANK PISCOTTA, PHD
Staff Scientist, Drug Discovery
Lieber Institute for Brain Development
frank.piscotta@libd.org

Project: Discovery of dopamine D5 receptor positive allosteric modulators for cognitive enhancement

TONY E. REEVES, PHD
Assistant Professor, Internal Medicine-Molecular Medicine
Wake Forest School of Medicine
tereeves@wakehealth.edu

Project: Development of Novel Opioid Interventions using Nonhuman Primate Fetal Imaging to Assess PD/PK and Efficacy

MARIO DE LA FUENTE REVENGA, PHARMD, PHD
T32 postdoctoral fellow, Virginia Institute of Psychiatric Genetics
Virginia Commonwealth University
fuente.revenga@gmail.com

Project: Beyond the boundaries of the psychedelic drug space to treat anhedonia in depressed patients

T'AIR RIGGINS, PHD
Postdoctoral Researcher,
Case Western Reserve University
ter48@case.edu

Project: Neurotechnology Design Features' Impact on the Identity and Function of Reactive Astrocytes

TRISTAN T. SANDS, MD, PHD
Assistant Professor, Neurology
Columbia University Medical Center
tts27@cumc.columbia.edu

Project: Precision Medicine for KCNQ3 Gain-of-Function Disease

PARTICIPANTS

2022 Course Participants

MOHAMMAD SHAHNAWAZ, PHD
Assistant Professor, Mitchell Center for Alzheimer's Disease and Related Brain Disorders
UT Health - McGovern Medical School

Mohammad.Shahnawaz@uth.tmc.edu

Project: Strain-specific conformational antibodies for immunotherapy of Synucleinopathies

MEHRDAD SHAMLOO, PHD
Professor, Neurosurgery
Stanford University - School of Medicine

mshamloo@stanford.edu

Project: Stroke, Drug Addiction

ANNA W. SROMEK, PHD
Research Associate Professor, Pharmaceutical Sciences
Northeastern University, Center for Drug Discovery

a.sromek@northeastern.edu

Project: Development of a Selective Agonist for the A2A-D2 Heterodimer for Treatment of Cocaine Use Disorder

OLIVIA TONG, MD
Assistant Professor, Neurology
University of California, Davis

octong@ucdavis.edu

Project: Chimeric autoantibody receptors (CAAR) T cells: a novel antigen -specific therapy for anti-muscle specific kinase (MuSK) myasthenia gravis

JIA BEI WANG, PHD
Professor, Pharmaceutical Sciences
School of Pharmacy, University of Maryland Baltimore

jwang@rx.umaryland.edu

Project: Study of Deuterated l-tetrahydropalmatine (l-THP), a Promising Non-Opioid Agent for Treatment of Pain and Opioid Use Disorder

ROBYN WISEMAN
Graduate Student, Pharmacology and Molecular Sciences
Johns Hopkins University

rwisema5@jhmi.edu

Project: GCPII inhibition for the treatment of cognitive deficits

PARTICIPANTS

2022 Course Participants

LEI YU, PHD
Distinguished Professor, Genetics
Rutgers University

yu@biology.rutgers.edu

Project: Antisense oligonucleotides (ASOs) as neurotherapeutics for chronic neuropathic pain

WEIQUN YU, MD, PHD
Assistant Professor, Medicine
Beth Israel Deaconess Medical Center

wyu2@bidmc.harvard.edu

Project: Targeting Cav1.2 to treat bladder dysfunction

ALEXANDER G. ZESTOS, PHD
Assistant Professor, Chemistry and Center for Neuroscience and Behavior
American University

zestos@american.edu

Project: Synthesis and Development of Novel PKC Inhibitors as Therapeutics for Amphetamine Abuse

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