

The Theory and Practice of Small-Molecule Drug Discovery

Session Date, Time	Topic and Description	Speaker Bios
July 10 th 4 - 4:45 pm At UCSD	Overview – From Concept to Market. We will give an overview of the disciplines involved in drug discovery and discuss the timelines and stages for programs on marketed drug examples. You will learn about the scientific challenges we face at every turn and the time and cost investments needed to make a drug discovery project successful.	<i>Yvonne Schmidt</i> is a medicinal chemist at Vertex in San Diego where she currently leads the outreach program. Previous to Vertex, she was a Humboldt postdoctoral fellow at the University of California, Irvine. Yvonne obtained her diploma (master’s degree) in chemistry and her PhD in organic chemistry at the Albert Ludwigs University in Freiburg, Germany. <i>Yoshi Ishihara</i> was born in Japan but raised in Canada, where he obtained his BSc and MSc in chemistry. He then moved to San Diego for his PhD studies in organic chemistry at Scripps Research. He has been at Vertex for 4 years as a medicinal chemist, working on cystic fibrosis research.
July 10 th 5:15 – 6 pm At UCSD	How do drug features contribute to the binding to a biological target? The seminar will provide a basic understanding of most important molecular interactions crucial for all living systems and how they contribute to the binding of a ligand to a receptor. We will also learn how structural features of a small molecule contribute to the binding.	<i>Anton Gulevich</i> has PhD in Organic chemistry and 4 years of drug discovery experience at Vertex working on both Hit-to-lead and Lead Optimization programs. Anton was trained as synthetic chemist with expertise in heterocyclic chemistry, C-H functionalization and multicomponent reactions. He is interested in new technologies facilitating drug discovery and efficient synthesis.
July 17 th 4 - 4:45 pm At UCSD	Target selection and essentials of Medicinal Chemistry. The seminar will discuss the importance of target selection in drug discovery and present common strategies used to identify agents with therapeutic utility. Fundamental concepts governing the discipline of medicinal chemistry will also be discussed and exemplified in a study case.	<i>Adam Scharf</i> obtained his BSc and PhD in organometallic chemistry from Tel-Aviv University, Israel. Prior to his work as a medicinal chemist at Vertex, Adam pursued postdoctoral training in the fields of bioinorganic chemistry and natural product synthesis at MIT and Boston University. He is currently interested in applications of automation technology to drug discovery programs.
July 17 th 5:15 – 6 pm At UCSD	Assay design, high-throughput screening & secondary follow-up assays. This seminar will discuss biological assays suitable to detect active substances in very large compound collections, determine their potency and selectivity and their suitability as starting points for optimization. In the second part of the lecture we will discuss the importance of secondary assays to further characterize the pharmacology of lead compounds with respect to their mechanism of action, their efficacy in cell systems and their selectivity against other biological targets that may result in undesirable effects.	<i>Brooke Grandinetti</i> is a research scientist at Vertex San Diego where she is working in the high throughput assays group. The group is responsible for validating and running high throughput screens to support all stages of drug discovery programs at Vertex. She received her Ph.D. from Fordham University in cell and molecular biology, and pursued postdoctoral work in chromatin biology at NYU. Prior to joining Vertex 5 years ago, Brooke previously held positions at Novartis and Shire, working both in high throughput screening groups as well as in target validation groups. <i>Tim Young</i> is a research scientist at Vertex San Diego where he is responsible for the biology of the Cystic Fibrosis program. Tim’s contributions to CF research have supported the nomination of six clinical candidates. Two of which have recently demonstrated positive phase III results in people with CF. Tim has over 20 years’ experience in drug discovery as a pharmacologist and project leader with previous positions held at Pfizer and GSK in research areas including neuroscience and infectious disease.
July 24 th 4 - 4:45 pm At UCSD	What does the body do to the drug? Preclinical pharmacokinetics; This session will give an overview of the key principles of DMPK -Drug M etabolism and P harmacokinetics as well as practical aspects of understanding the ADME (A bsorption, D istribution, M etabolism, E xcretion) properties of potential new medicines using <i>in vivo models and in vitro</i> tools.	<i>Mike Wester</i> leads the High Throughput In Vitro ADME team at Vertex, San Diego. This team provides decision driving data to global project teams. Prior to joining Vertex, Mike held positions at AstraZeneca, Sweden and Pfizer, La Jolla. Mike obtained his PhD at the University of New Mexico and was a postdoctoral fellow at The Scripps Research Institute. <i>Julie Laterreur</i> is a DMPK scientist at Vertex San Diego prior to Vertex she obtained her BSc and MSc in Bio-Chemistry in Canada. Julie has been working for Vertex for more than 10 years working as DMPK representative for lead optimization programs and development stage assets.
July 24 th 5:15 – 6 pm At UCSD	How do we get the Drug on board (into blood circulation)? Drug formulation. In order for a drug to achieve its desired effect, two things must occur which involve the drug 1) travelling through the body to the site of action, and 2) reaching the target at an efficacious concentration. To achieve these goals, pharmaceutical scientists devise formulations of the drug to bolster delivery or influence release profiles. In this presentation, an overview of formulation principles used for common routes of administration will be presented. You will also learn about formulation strategies used in industry.	<i>Tim Alcacio</i> is a formulation scientist and has been with Vertex in San Diego for the past 12 years. Prior to joining Vertex, he worked at Pfizer La Jolla, where he was involved in studying the solid state of proprietary compounds for oncology therapies. He also initiated and lead the sites’ the small molecule crystallography efforts. Tim obtained his BS from UC Davis, and Ph.D. from North Carolina State University.
July 31 st 4 - 4:45 pm At UCSD	What does the drug do to the body? Preclinical pharmacology. So, we have a test compound that can be delivered to target tissue. Now what? Here, we will review questions that can be addressed using preclinical animal models (efficacy assessment), as well as fundamental principles regarding proper model selection, experimental execution, and data interpretation.	<i>Dennis Fiorino</i> , is a Research Fellow and biologist at Vertex Pharmaceutical Inc., where he studies the pharmacology of pain and cystic fibrosis. He obtained his doctoral degree in Neuroscience from the University of British Columbia. Prior to joining Vertex, Dennis held positions at Eisai Co., Ltd (Tsukuba, Japan) and Memory Pharmaceuticals Corp. (New Jersey) where he contributed to drug discovery programs for psychiatric and cognitive disorders.
July 31 st 5:15 – 6 pm At UCSD	How do we determine a drug’s safety early? Exploratory toxicology. The purpose of this lecture is to gain a familiarity with the fundamentals of toxicology and approaches to safety assessment during drug development. We will cover both classic approaches to characterize target organ toxicities as well as emerging mechanistic tools to understand systems toxicology.	<i>Jeffrey A. Willy</i> , is a Research Fellow in Discovery and Investigative Toxicology at Vertex Pharmaceuticals in San Diego, CA. Jeff received his PhD. from the Indiana University School of Medicine (IUSM) where he studied the role of the unfolded protein response during the pathogenesis of nonalcoholic steatohepatitis. Jeff also holds an adjunct faculty position at IUSM where he is a graduate student advisor. Jeff worked at Eli Lilly for over a decade, where he ran a lab focused on mechanistic and systems toxicology that incorporated novel applications of CRISPR/Cas9. Jeff has received multiple awards, including a 2016 SOT Young Investigator Award, a 2016 MBSS SOT Research Award, 2015 AASLD Basic Science Young Investigator Award, to name a few. Jeff also holds a music degree in percussion from the Eastman School of Music and teaches high school students in his free time.
August 7 th 4 - 4:45 pm At Vertex	Putting it all together. The discovery of Orkambi.	<i>Mark Miller</i> earned his BS at SUNY College at Buffalo and his PhD at the University of California at San Diego. He is a Director of Medicinal Chemistry at Vertex Pharmaceuticals in San Diego with over 18 years of drug discovery experience and a co-inventor of two FDA approved drugs to treat Cystic Fibrosis (lumacaftor, tezacaftor).
August 7 th 4:45 - 6 pm	A tour of the Drug Discovery facility at Vertex Pharmaceuticals, San Diego.	