



**UNITED** Scientific  
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# 5<sup>TH</sup> INTERNATIONAL CONFERENCE ON DRUG DISCOVERY DEVELOPMENT AND LEAD OPTIMIZATION

*Emerging Disease Target Validation in Metabolism,  
Infectious Diseases, Cancer and Cannabis*

## Venue

Crowne Plaza Boston - Newton  
320 Washington St, Newton, MA 02458

# DRUG DISCOVERY-2019

November 21-23, 2019

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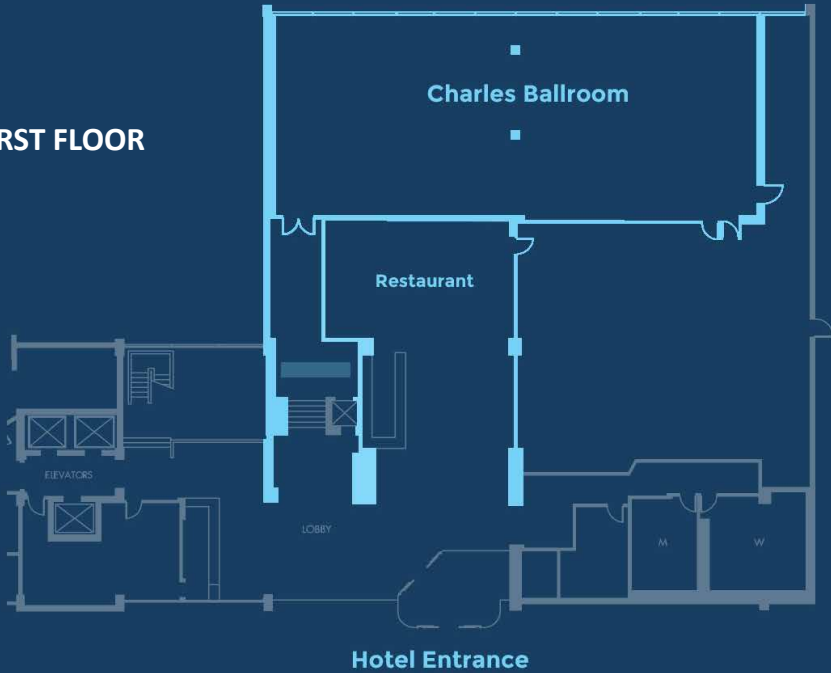


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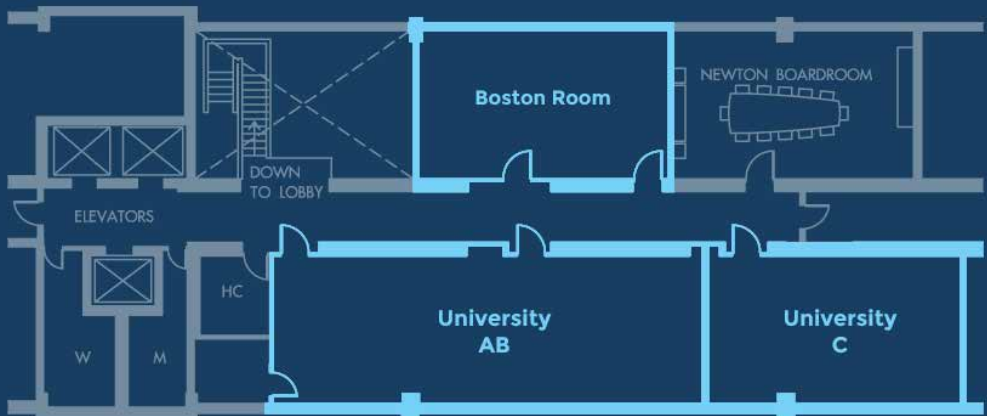


# Venue Floor Plan

## FIRST FLOOR



## SECOND FLOOR



## Welcome Message

*It is my great pleasure as organizer and chairman to welcome everyone to the 2019, 5<sup>th</sup> International Drug Discovery Conference. I would like to begin by acknowledging Dr. Troels Koch, VP & Head of Research, RNA Therapeutics Roche Innovation Center Copenhagen Denmark as co-organizer and Mr. Satish Noolu as meeting coordinator from United Scientific Group for their critical input and assistance in organizing this meeting and website as well as generous sponsor support from Covance and Synthego. I would also like to thank the city Boston for hosting our meeting with their exceptional accommodations in the backdrop of this historic city and its cultural and high-tech ambiance. This year's conference emphasizes cutting-edge research and preclinical development being conducted globally by companies and contract research and academic laboratories. The goal of this conference series continues to be to encourage a dialog on ideation, collaboration and resource sharing. This three-day conference is structured around Plenary and Keynote addresses, five symposia and a poster session. Our plenary presentation by Dr. Carl Dieffenbach, Director of DAIDS, NIAD at the National Institutes of Health will evaluate the almost 40 year drug discovery effort in the battle to end HIV. Following the morning plenary presentation on the first day, the conference will dive into five symposia consisting of scientists and opinion leaders on the topics of: 1. emergent drug development approaches and opportunities, 2. emerging drug development for cannabis, 3. nucleic acids as drugs, drug targets and gene editing tools, 4. drug development for infectious disease and immune modulation and 5. oncology drug target validation. The range of topics covered by speakers in each symposium will be lead by keynote presentations from Drs. Lawrence Verneti, Director of Early Stage Drug Safety, University of Pittsburgh, Ethan Russo, Director of R&D, International Cannabis and Cannabinoid Institute, Bo Rode Hansen, President and CEO of Genevant Sciences, Paul Dunman, Professor at the University of Rochester, School of Medicine and Dentistry and Ives Pommier, Chief for Developmental Therapeutics, NIH. In these keynote addresses we will learn of diverse critical paths that can be taken in drug discovery and lead optimization in both established drug discovery areas as well as emerging opportunities. The meeting plan is to afford amply time for informal networking, brain storming and dynamic conversations over posters. I look forward to meeting you and sharing our collective talents at this very exciting, informative and interactive conference.*



A handwritten signature in dark ink that reads 'Harold C. Smith'.

**Harold C. Smith, Ph.D**

**Conference Chairman - Drug Discovery-2019**

*Department of Biochemistry and Biophysics  
Medical Center, Cancer Center and Center for RNA Biology  
University of Rochester, School of Medicine and Dentistry  
Rochester, NY*

## Conference Chairman

*Harold C. Smith, Ph.D*

*Conference Chairman - Drug Discovery-2019*

*Department of Biochemistry and Biophysics  
Medical Center, Cancer Center and Center for RNA Biology  
University of Rochester, School of Medicine and Dentistry  
Rochester, NY*



*Dr. Smith earned his M.A. and Ph.D. from SUNY at Buffalo, following earning his B.S. and M.S. degrees from Purdue University. His postdoctoral research was conducted at Baylor College of Medicine. He is a full Professor in Biochemistry, Biophysics at the University of Rochester, School of Dentistry and Medicine, and is a Member of the University's Cancer Center and Center for RNA Biology. Dr. Smith's research has been supported through grants from the NIH and Bill and Melinda Gates Foundation, the United States Air Force and the Office of Naval Research. He is the author of more than 141 peer-reviewed manuscripts and reviews during his career, exceeding more than 9,973 citations. He is a member of the RNA Society, American Society for Biochemistry and Molecular Biology and International Society for Antiviral Research. He received the SUNY at Buffalo's Distinguished Alumni Award as well as several teaching and mentoring awards from the University of Rochester. Dr. Smith is an opinion leader in RNA and DNA editing. He established the first in an ongoing Gordon Research Conference series on RNA and DNA Editing and Modification. In 2003, Dr. Smith founded OyaGen, Inc. The company's current leads are in preclinical development as first-in-class antiviral therapeutics for HIV and Ebola. In 2016, Dr. Smith founded CannaMetrix, LLC with the goal of establishing human cell-based, high throughput assays to quantify cannabis product potency and consistency using biological standards*

## Conference Co-Chairman

*Troels Koch, M.Sc., Ph.D.*

*Conference Co-Chairman - Drug Discovery 2019*

*VP & Head of Research, RNA Therapeutics*

*Roche Innovation Center Copenhagen*

*Denmark*



*Troels Koch (TK) has 20 years' experience in the international life science and biopharmaceutical industry. Founder of several biotech companies of which Exiqon A/S and Santaris Pharma A/S are the most commonly known. Santaris Pharma A/S was successfully exited in August 2014 – acquired by Roche, and Exiqon A/S was acquired in 2016 by Qiagen. TK has been positioned in company management teams and taken part in all aspects of executive decision making: Company strategy, VC fundraising, deal makings, partnering, IP strategy & prosecution, drug discovery and R&D. TK pioneered LNA therapeutics and has been responsible for keeping LNA antisense science and technology at an international lead position. He has worked with RNA therapeutics for 20 years and taken an active role in all steps of oligonucleotide drug discovery and development. TK has built R&D organizations up to 75 co-workers, held 100+ invited presentations at international conferences and is author of 80+ peer reviewed publications.*

## SILVER SPONSOR



# EARLY PHASE DEVELOPMENT SOLUTIONS

Making every day of your journey count

With so many different regulatory requirements and scientific challenges in the early stages of drug development, it can be difficult to navigate a way forward, easily. Discover a clear path with Early Phase Development Solutions. It's a programmatic approach for meeting your drug development goals that teams you up with drug development leaders to prospectively plan and execute the required studies in an expedited way. By making every day of the journey count, Early Phase Development Solutions has helped hundreds of biotech firms reach their program goal up to 30 percent faster over the past three years.

### BEGIN WITH THE END IN SIGHT

#### Start off on the right track

Your vision for your molecule defines your entire program. From your development team and strategy to your contracts and financing, everything is customizable to your requirements.

#### Stay on course

Keep up to date on the details and progress of your program with regular status updates on your program timeline and proactive reports on potential risks to your candidate's development.

#### Achieve your program milestone

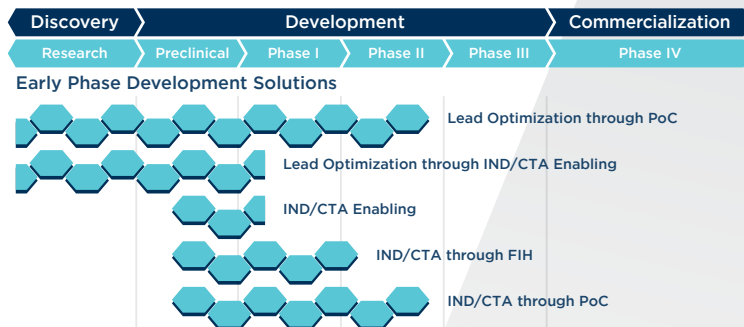
Reach your clinical goal faster with an approach that eliminates white space in your early drug development timeline and streamlines your transition from nonclinical to clinical development.

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SOLUTIONS MADE REAL<sup>®</sup>



# HOW FAR YOU GO IS UP TO YOU

Whether you plan to complete an IND/CTA-enabling program or you need to gain the clinical insight that a first-in-human (FIH) or proof-of-concept (PoC) study can provide, you can enjoy the journey with a dedicated team and a singular, cohesive strategy.



## Real-world benefits of a time-tested approach

Join more than 225 biotech ventures that have experienced the real-world benefits of a programmatic approach over the course of the past three years.

### PARTNERSHIP

**A dedicated team that's with you for the entire journey**

Collaborate with a Drug Development Leader and a Certified Project Manager at the helm of your program

Gain specialized expertise in nonclinical, clinical and regulatory, as you need it

De-risk your molecule development with a team that proactively monitors your program

### CONTINUITY

**A proactive strategy, from start to finish**

Get an integrated development plan that minimizes time between studies

Stay up to date through regular communications with your team

Transition seamlessly from nonclinical into clinical development

### VALUE

**An integrated and streamlined approach that adds value to your asset**

See your estimated total program timeline and cost up front

Select a flexible financial model that fits your goals

Reach critical milestone dates on time

Obtain valuable clinical insight by taking your program all the way through FIH or PoC

**READY TO ACCELERATE YOUR JOURNEY THROUGH DRUG DEVELOPMENT?  
TAKE THE CLEAR PATH FORWARD WITH EARLY PHASE DEVELOPMENT SOLUTIONS.**

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8:00 - 9:00 Registrations

09:00 - 09:10 Introduction and Welcome address by Conference Chairman - Prof. Harold Smith, Rochester University, New York, NY

**Plenary Talk** Talk: 30 Min | Q&A: 10 Min

**Carl Dieffenbach** **09:10**  
 National Institutes of Health, Bethesda, MD

**Title: Drug Development for HIV: We Have Come So Far, and We Are Not Yet Done**



Carl W. Dieffenbach, Ph.D., serves as Director of the Division of AIDS (DAIDS). Dr. Dieffenbach oversees a global HIV/AIDS research portfolio of more than \$1 billion and a staff of more than 150 federal employees. He played a key role in restructuring the DAIDS-supported clinical trials research networks and has actively fostered collaboration and partnerships with other federal agencies, international research organizations, foundations, and industry. In 1992, he joined DAIDS as the chief of the preclinical therapeutics group. Upon his appointment, Dr. Dieffenbach spearheaded important research initiatives that accelerated the progress of basic research on HIV pathogenesis and directly resulted in new clinical studies of novel AIDS therapies. In 1996, he was promoted to Director of the DAIDS Basic Sciences Program, where he remained until being selected as the division director in 2008. In 2011, Dr. Dieffenbach received the Distinguished Alumnus Award of the Department of Chemistry and Biochemistry, University of Maryland.

09:50 Refreshment Break

**Symposium I: Emergent Drug Development Approaches and Opportunities**

**Co-Chairman:** *Lawrence Vernetti, University of Pittsburgh, Pittsburgh, PA*  
*Marina Seme-Nelson, Covance Inc., Madison, WI*

**Keynote Talk** (Talk: 25 Min | Q&A: 5 Min)

**Lawrence Vernetti** **10:10**  
 University of Pittsburgh, Pittsburgh, PA

**Title: Non-Alcoholic Fatty Liver Disease (NAFLD) Drug Discovery Through the Application of Quantitative Systems Pharmacology and Microphysiology Systems**



Lawrence Vernetti, Ph.D. currently working as Research Associate Professor in the department of Computational and Systems Biology and he is the Director of Early Drug Safety, University of Pittsburgh Drug Discovery Institute, PA. Before joining University of Pittsburgh he worked as Director of Cellular Toxicology at Cellumen from 2007-2010 and as a Research Scientist at Abbott Laboratories during 1992-2007. He completed his Ph.D in the year 1992 from University of Arizona, Tucson, AZ. His research focus is in the application of early drug safety testing using a variety of in vitro, in vivo and in silico methodologies.

**Symposium Talks** (Talk: 20 Min | Q&A: 5 Min)

10:40 **Marina Seme-Nelson, Covance Inc., Madison, WI**  
**Bridging the Gap: IND to FIH and Beyond**

- 11:05 **Stephanie C. Oestreich**, EVP, Head BRIDGEs Partnerships North America, Evotec  
**Path to IND – How to Use Partners to Create Valuable Data for a Startup**
- 11:30 **Adam Simon**, Qulab Inc., Los Angeles, CA  
**AI-Automated De Novo Small Molecule Design**
- 11:55 **Ravindra Peravali**, Karlsruhe Institute of Technology, Germany  
**Using Zebrafish Embryonic and Larval Behavior for Drug Discovery and Testing**
- 12:20 **Michael Graziano**, TARA Biosystems, Inc., New York, NY  
**Cardiotype SM Engineered Cardiac Tissues: An iPSC Derived Human-Relevant Model for Drug Discovery and Development**

**12:45 Lunch & Networking**

- 13:45 **Robert Ricciardi**, University of Pennsylvania, PA  
**Developing a Novel Drug to Treat Herpes Ocular Keratitis, the Leading Cause of Ocular Blindness in the World**
- 14:10 **TBA**  
**TBA**

**Short Talk** (Talk: 12 Min | Q&A: 3 Min)

- 14:35 **Pradeep Karla**, Howard University, Washington, D.C.  
**Drug Efflux Transporters - Targets for HIV Drug Delivery?**
- 14:50 **Helen Yu**, Centre for Advanced Studies in Biomedical Innovation Law, University of Copenhagen, Denmark  
**Leveraging Research Failures to Accelerate Drug Discovery and Development**

**Symposium II: Emerging Drug Development for Cannabis**

**Co-Chairman** *Ethan Russo, International Cannabis and Cannabinoids Institute, Czech Republic, EU*  
*Dana M. Lambert, Andira Pharmaceuticals, Vancouver, Canada*

**Keynote Talk** (Talk: 25 Min | Q&A: 5 Min)

**Ethan Russo** **15:05**  
International Cannabis and Cannabinoids Institute, Czech Republic, EU

**Title: Pharmacology of Cannabis, a Whirlwind Tour**



**Ethan Russo, MD**, is a board-certified neurologist, psychopharmacology researcher, and Director of Research and Development of the International Cannabis and Cannabinoids Institute (ICCI) based in Prague, Czech Republic. Previously, he was Medical Director of PHYTECS (2015-2017) and from 2003-2014, he served as Senior Medical Advisor, medical monitor and study physician to GW Pharmaceuticals, United Kingdom. He graduated from the University of Pennsylvania (Psychology), and the University of Massachusetts Medical School, before residencies in Pediatrics in Phoenix, Arizona and in Child and Adult Neurology at the University of Washington in Seattle. He is a Past-President of the International Cannabinoid Research Society and is former Chairman of the International Association for Cannabinoid Medicines. He serves on the Scientific Advisory Board for the American Botanical Council. He has also published numerous book chapters, and over 50 articles in neurology, pain management, cannabis, and ethnobotany. He has consulted or lectured in 39 US states and Canadian provinces and 39 countries.





- P-10 [Ibrahim M. Moustafa](#), The Pennsylvania State University, PA  
**Detection of Novel Small-Molecule Binding Site on the Viral Capsid Protein VP3 of Coxsackievirus B3 and Identification of Candidate Inhibitors by Virtual Screening**
- P-11 [Joel Frandsen](#), University of Nebraska Medical Center, Omaha, Nebraska  
**Morin Derivatives on Neural Glyoxalase Pathway Enhancement in an Oxidative Stress Model of Alzheimer's Disease**
- P-12 [Prasanthi Medarametla](#), University of Eastern Finland, Helsinki, Finland  
**Computational Approaches in Identification of Lsrk Inhibitors Targeting Quorum Sensing**
- P-13 [Lisa L. Hester](#), Flowered Wellness, LLC., Orchard Park, NY  
**Bridging the Gap; A Proposal to Facilitate and Accelerate the Transition of Cannabis Research to Clinical Practice**
- P-14 [Cynthia Lichorowic](#), Aerie Pharmaceuticals, Inc., Durham, NC  
**Synthesis and Development of New, Potent JAK Inhibitors for the Treatment of Ocular Indications**
- P-15 [Marie Wiik](#), Recipharm OT Chemistry, Uppsala, Sweden  
**Successful Collaboration between Recipharm OT Chemistry and Oncopeptides AB in the Development of Melflufen, a Peptidase-Potentiated Alkylating Agent in Clinical Trials**

**Short Talks & Poster Slots Available**

**Symposium III: Nucleic Acids as Drugs, Drug Targets and Gene Editing Tools**

**Co-Chairman** *Troels Koch, Roche Innovation Center Copenhagen, Denmark*  
*Bo Rode Hansen, Genevant Sciences, Boston, MA*

**Keynote Talk** (Talk: 25 Min | Q&A: 5 Min)

**Bo Rode Hansen** **08:30**  
 Genevant Sciences, Boston, MA

**Title: TBA**



**Bo Rode Hansen**, MBA, PhD joined Genevant in October 2018, taking on the roles of President and Chief Executive Officer in November 2018. Previously, Bo served as Global Head of RNA Therapeutics and General Manager of the Roche Innovation Center Copenhagen. Prior to Roche, he was Executive Vice President and Head of Drug Discovery & Alliance Management at Santaris Pharma (acquired by Roche). He is a Pioneer in RNA therapeutics and has led multiple discovery programs translating into clinical candidates in the areas of oncology, infectious diseases, rare genetic-, neurological- and metabolic disorders. He holds a PhD from Kobenhavns Universitet, an MS in Biochemistry from the University of Copenhagen and a BS from Universita Degli Studi di Pavia.

**Brian Schneider** **09:00**  
 Synthego, Redwood City, CA

**Title: Optimizing CRISPR Gene Editing Tools for Drug Discovery**



**Brian Schneider**, Ph.D is the Senior Territory Account Manager at Synthego Corporation, Boston, MA. Before joining Synthego Inc., he worked as Director, Strategic Accounts at Gen9 In., from 2015-16, and as Area Business Director at CyVek during 2014 and as Corporate Account Manager at Roche from 2011-2014. He graduated from Massachusetts Institute of Technology (MIT) in 1999.

**09:30 Refreshment Break**

**Symposium Talks** (Talk: 20 Min | Q&A: 5 Min)

- 09:50 **Sven Korte**, Covance Preclinical Services GmbH, Münster, Germany  
**Conduct and Design of Nonhuman Primate Studies for First in Man (FIM) and beyond. Oligos: Non Systemic Delivery – Rare and Orphan**
- 10:15 **Alexander McCampbell**, Biogen, Boston, MA  
**Antisense Oligonucleotides as an Emerging Modality for the Treatment of Neuromuscular Diseases**
- 10:40 **Søren Warming**, Genentech, Inc., San Francisco, CA  
**CRISPR Off-Target Analysis in Genetically Engineered Rodents**
- 11:05 **Norbert Pardi**, University of Pennsylvania, Philadelphia, PA  
**Development of New Generation Vaccines using Nucleoside-Modified mRNA**

- 11:30 **David Butler**, Wave Life Sciences, Medford, MA  
**Control of Backbone Stereochemistry Provides a New Dimension for the Optimization of Oligonucleotide Drug Candidates**
- 11:55 **David Evans**, Sirnaomics, Inc., Cambridge, MA  
**Discovery and Development of the Novel RNAi Therapeutics to Treat Cancer and Fibrosis**
- 12:20 **Andrea Kasinski**, Purdue University, West Lafayette, IN  
**Ligand-Conjugated Delivery of Therapeutic microRNAs**

**12:45 Lunch & Networking**

**Symposium IVa: Drug Development for Infectious Disease and Immune Modulation**

Co-Chairman *Paul Dunman*, University of Rochester Medical Center, Rochester, NY  
*Anna Jacobs*, Walter Reed Army Institute of Research, Silver Spring, MD

**Keynote Talk** (Talk: 25 Min | Q&A: 5 Min)

**Paul Dunman** **13:45**  
 University of Rochester Medical Center, Rochester, NY

**Title: TBA**



**Paul Dunman**, Ph.D. is the Principal Investigator in the School of Medicine and Dentistry and Associate Professor in the Department of Microbiology and Immunology (SMD) at University of Rochester Medical Center, Rochester, NY. He completed his Ph.D from University of Medicine and Dentistry-NJ (UMDNJ) in 1999 and Post-doctoral studies in the year 2001. He has received Young Investigator of the Year award in 2003a and Distinguished Investigator award in 2010 from University of Nebraska Medical Center.

**Symposium Talks** (Talk: 20 Min | Q&A: 5 Min)

- 14:15 **Shuji Ogino**, Harvard Medical School, Boston, MA  
**Integrative Analyses of Environment, Microbiome, Genomics, and Immunity for Precision Medicine**
- 14:40 **Thomas Webster**, Northeastern University, Boston, MA  
**Are Nanoparticles Suitable Drug Substitutes ? Fighting Infection, Inhibiting Cancer, and Growing Tissues**
- 15:05 **Anna Jacobs**, Walter Reed Army Institute of Research, Silver Spring, MD  
**TBA**
- 15:30 **Rachel Wozniak**, University of Rochester Medical Center, NY  
**Development of a Broad Spectrum Antimicrobial Combination for the Treatment of Ocular Infections**

**15:55 Refreshment Break**

- 16:15 [Cynthia Dowd](#), George Washington University, Washington, DC  
**MEPicides as Promising Antimicrobials**
- 16:40 [Yan Yuan](#), University of Pennsylvania, Philadelphia, PA  
**An mTORC2 Inhibitor Reveals A Crucial Role of mTORC2 Signaling in Epstein-Barr Virus Lytic Replication and Tumorigenesis**
- 17:05 [Mu Yuguang](#), Nanyang Technological University, Singapore  
**Binding Modes of Teixobactin to Lipid II: Molecular Dynamics Study**

**Short Talks & Poster Slots Available**









*We wish to see you  
at  
Drug Discovery-2020*



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