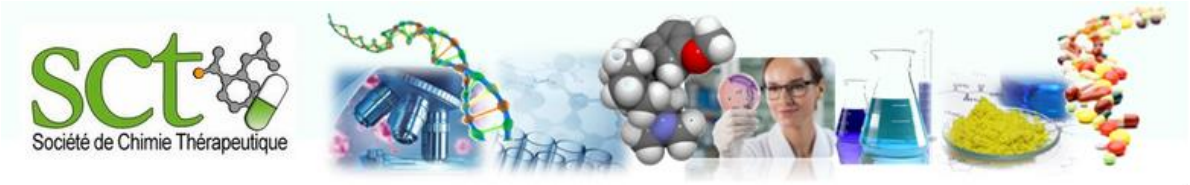
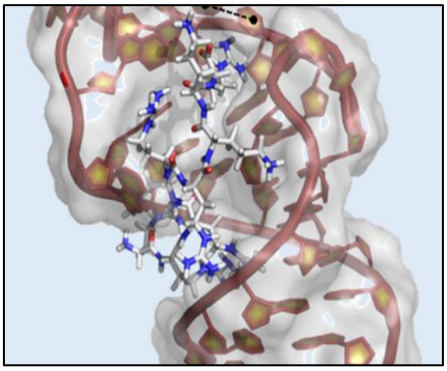


Drug Discovery in the RNA world



SAVE THE DATE: **December 11th, 2019, Paris, France**

The French Medicinal Chemistry Society (Société de Chimie Thérapeutique – SCT) is pleased to invite you to Paris, on December 11th, 2019 for the 4th one-day thematic symposium focusing on the contribution of Chemical Biology to Molecular Therapeutic Innovations.

Previous symposia were:

- Chemical Biology: Contribution to Molecular Therapeutic Innovation. - A New Role for Chemistry ? (November 26th, 2013).

- Chemical Biology: Bioorthogonal Chemistry contributing to Molecular Therapeutic Innovation (April 8th, 2015)

- Chemical Biology: Contribution to Molecular Therapeutic Innovation - Conjugates and Drug Discovery Chemistry: new challenges for targeted therapies – (December 7th, 2017)

The meeting will take place at the "Maison des Associations de Solidarité", 10/18 rue des terres au curé - 75013 PARIS – France

Link for registration :

http://www2.sct-asso.fr/form.php?langue=english&cle_menus=1187970249

Aim of the symposium:

The aim of the symposium is to illustrate the great potential and applications of RNA-targeting modalities in contributing to therapeutic innovation.

This meeting is dedicated to a large audience of organic and medicinal chemists, biochemists and biologists from academic and industry. During this day, we will focus on recent advances in the field of RNA targeting using small molecules as well as oligonucleotides: the synthetic

challenges involved in the design of RNA-targeting agents and on the validation of relevant RNA targets, as well as on the current methodologies used to study RNA/ligands interactions. RNA has already been drugged. Ribosomal RNA was drugged serendipitously with natural product antibiotics and later linezolid. A design-driven approach to drugging RNA was achieved with antisense oligonucleotides (ASOs) and then by RNA interference (RNAi) mechanisms.

More recently, small molecules that bind to the FMN riboswitch and to SMN2 pre-mRNA were reported and are close to clinical application. All those recent developments in the field of RNA-targeting definitely hold promise for future therapeutic applications.

List of confirmed speakers to date :

Matthew DISNEY (Scripps, Florida, USA) : **"translating RNA sequence into lead small molecule medicines."**

Matthew DISNEY is currently a Professor in the bi-coastal Department of Chemistry at The Scripps Research Institute. His laboratory is focused on understanding RNA-ligand interactions and using this information to rationally design small molecules that modulate and study RNA function or toxicity from only sequence. He received his early schooling in the Baltimore Catholic School System, his B.S. from the University of Maryland in Chemistry, his Ph.D. from the University of Rochester in Physical Chemistry with Doug Turner. The group's work has garnered various awards including the Sackler Prize, Barry Cohen Award in Medicinal Chemistry, the NIH Director's Pioneer Award, and the Tetrahedron Young Investigator Award.

Maria DUCA (Université Côte d'Azur, France) : **"Synthetic small-molecule RNA ligands : Scope and applications"**

Maria DUCA is head of « Targeting of Nucleic Acids » research group in the Institute of Chemistry of Nice (Université Côte d'Azur - CNRS). After undergraduate studies in Pharmacy and Medicinal Chemistry (Faculty of Pharmacy, Bologna, Italy), she obtained her PhD in Molecular Biochemistry under the supervision of Dr. Paola B. Arimondo (National History Museum, Paris, France) working on topoisomerase II inhibitors targeting specific DNA sequences. A 2- year post-doctoral training in Sydney Hecht's lab (Departement of Chemistry, University of Virginia, USA) allowed her to pursue the study of nucleic acids/small molecules interactions working on targeted protein mutagenesis upon tRNA chemical modification. After CNRS recruitment as a Research Scientist in 2007, her research activities focus on the targeting of non-coding RNAs using synthetic small molecules toward innovative therapeutic approaches both in anticancer and antimicrobial applications.

Mélanie ETHEVE-QUELQUEJEU (Université Paris Descartes, France) : **"Modified RNAs as Molecular Tools for Structural and Functional Studies of RNAs dependents Enzymes"**

Mélanie ETHEVE-QUELQUEJEU is professor of chemistry at Paris Descartes University. She leads the group "Synthesis of RNAs, Nucleosides, Peptides and Heterocycles", in the laboratory "Chimie & Biochimie, Pharmacologiques et toxicologiques", UMR 8601. She obtained her PhD at the University UPMC, Paris VI, in France in 1997. She conducted postdoctoral studies first at Stanford University in California with Prof. J.P. Collman, and then at Santa Barbara University with Prof. B. Lipshutz. In September 1999, she was appointed Assistant Professor in the Prof. J. M. Valery's group at the UPMC. She obtained a full Professor position at Paris Descartes University in 2012. She works in the field of nucleosides and nucleotides chemistry, on chemical biology of RNAs, and she developed projects on synthesis of β-lactams, peptides and heterocycles.

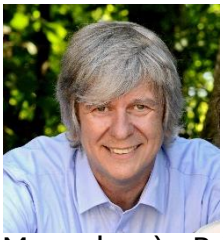
Eric ENNIFAR (Université de Strasbourg, France) : **"Structure-guided discovery of novel ligands targeting the HIV-1 genomic viral RNA"**

Eric ENNIFAR received in 1999 a Master degree in Biological Crystallography and NMR from University of Strasbourg. He obtained a Ph.D. in Structural Biology in 2001 from the University of Strasbourg working with Dr. P. Dumas. He then moved to the European Molecular Biology Laboratory in Heidelberg for a postdoc with Dr. D. Suck. In 2003, he joined the CNRS unit "Architecture et Réactivité de l'ARN" in Strasbourg as a CNRS Research Scientist. He is now CNRS Research Director, group leader of the team "Structure and Dynamics of Biomolecular Machines". He is also president of the French Biophysical Society and chief scientific officer of the IBMC Biophysical Platform since 2017. His current research is focused on the ribosome translational machinery and structure-based design of HIV-1 RNA ligands using structural and biophysical approaches. He was awarded the CNRS 2014 bronze medal.

Jonathan HALL (ETH, Switzerland) : **"A Bright Future for Oligonucleotide Drugs"**

Jonathan HALL received his Ph.D. in organic chemistry at Imperial College in London. He did post-doctoral work with J.-M. Lehn in Strasbourg (FR) and with Y. Kishi in Cambridge (USA). He joined the nucleic acids section at Novartis Pharmaceuticals in Basel in 1992. In 2003 he was presented with the Novartis Leading Scientist Award. In the following six years his group established high-throughput oligonucleotide synthesis and genome-wide screening using siRNAs. Together with colleagues from the neuroscience department they developed methods to use siRNAs *in vivo* which resulted in therapeutic effects of siRNAs in clinical models of neuropathic pain. Jonathan Hall became full professor for Pharmaceutical Chemistry at the ETHZ in 2007. From 2012-2014 he was chair of the Institute of Pharmaceutical Sciences. Jonathan Hall serves on the steering committee of the Drug Discovery Network Zurich (DDNZ), of which he is a co-founder. Currently, he manages a 15-member group comprising graduate students and post-doctoral fellows. The long-term objective of his group is to increase the drugability of RNA using new classes of oligonucleotide- and small molecule-ligands.

Gerhard MUELLER (Gotham Therapeutics, Germany) : **"Epitranscriptomic readers, writers, and erasers: a biophysics and medicinal chemistry perspective"**



Gerhard MUELLER is Chief Scientific Officer at Gotham Therapeutics, a transatlantic biotech company located in New York City, US, and Munich, Germany, focusing on epitranscriptomic drug discovery. In his capacity, Gerhard oversees several small-molecule drug discovery projects targeting mRNA modifying enzymes, as well as m6A reader proteins. Gerhard held several research management positions at biotech companies (Axxima Pharmaceuticals, GPC Biotech) and within the CRO industry (Proteros Fragments, Mercachem). Prior to that, Gerhard held a number of senior research and project management positions within the pharmaceutical industry, i.e. at Glaxo Group Research in Verona, Italy, at Bayer AG, Leverkusen, Germany, and he headed the medicinal chemistry unit at Organon N.:V. in Oss, NL. Gerhard obtained his PhD from the Technical University Munich, under the supervision of Prof. Dr. Horst Kessler establishing the research field of antiadhesive integrin antagonists.

Alleyn PLOWRIGHT (Sanofi-Aventis Deutschland GmbH, Germany) : **"Modulating RNA - recent drug discovery approaches and opportunities for diverse modalities"**

Alleyn PLOWRIGHT obtained his PhD in organic chemistry with Professor Gerald Pattenden at the University of Nottingham, UK in 1999, and continued with postdoctoral studies in chemical biology with Professor Andrew Myers at Harvard University, USA. In 2002 Alleyn joined AstraZeneca UK. In 2008 he moved to AstraZeneca Sweden where he took on the role of Associate Director Medicinal Chemistry and then in 2012 became Senior Principal Scientist and Project Leader in the Cardiovascular and Metabolic Diseases Innovative Medicines unit. In 2017 Alleyn moved to Sanofi as Head Integrated Drug Discovery Germany leading a cross-disciplinary drug discovery unit. His current research interests include Drug Design, Phenotypic Drug Discovery and diverse chemical approaches to treat metabolic, cardiovascular, immunological and rare diseases.

Helene TRAN (Servier Research Institute, France) : **"Antisense Oligonucleotides Therapeutics for CNS Disorders"**

Helene TRAN is a project leader in the Neuropsychiatry Center of Therapeutic Innovation at the Servier Research Institute since December 2018. After a master in Neurobiology at Imperial College London, she obtained her PhD in Molecular Neurobiology under the supervision of Dr. Nicolas Sergeant (INSERM, Lille) working on RNA mediated toxicity in myotonic dystrophies. She then joined the laboratory of Dr. Fen-Biao Gao at the University of Massachussetts Medical School (UMASS, MA USA) as a postdoctoral fellow to elucidate the disease mechanisms related to the most common genetic cause of amyotrophic lateral sclerosis using fly, mouse and patient derived neuronal cells. She then joined the UMASS Neurology department chaired by Dr. Robert Brown as a faculty member where she developed nucleic acid based therapeutics in collaboration with the RNA therapeutics institute (Dr. J Watts and A. Khvorova) and Wave Life Sciences.

Organizing committee

Dr. Amaury FERNANDEZ-MONTALVAN (Servier, France)
Dr. Yann FORICHER (Sanofi, France)
Dr. Brigitte LESUR (Vice-President SCT, France)
Pr. Sébastien PAPOT (President SCT, University of Poitiers, France)
Dr. Frédéric SCHMIDT (Curie Institute, Paris, France)

Fall One-day Symposium Secretariat
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