



UNLOCKING THE PREVIOUSLY “UNDRUGGABLE”

April 13th-14th, 2021 | Virtual | GMT Timezone

With an already ever-changing R&D landscape rocked by the effects of the pandemic, there’s never been a more crucial time to bring together the best minds from biotech, pharma and academia to unlock the next generation of drug targets. Fresh off the back of the U.S. Undruggable Leaders Forum, prepare to continue your learning journey with the crème de la crème of all things “undruggable”.

With an agenda completely dedicated to overcoming the key challenges uniting all modalities against intractable targets, a carefully-curated selection of crucial case studies, and a speaker faculty of top industry names, the Undruggable Leaders Forum Europe will enable attendees to learn from the experiences of industry experts and equip them with the tools to further their own “undruggable” pipeline.

Day 1: Tuesday, April 13th, 2021

8:00am **Conference Platform Opens**

The conference platform opens before the agenda starts, giving you time to plug the presentations, panels and discussions into your agenda for the rest of the day. This also gives you plenty of time to get familiar with the platform.

9:00am **Chair's Opening Remarks & Setting the Scene**

Mads Aaboe Jensen, External Innovation Officer, RNA Molecule Research, *Roche*

9:05am Keynote Presentation - **Hijacking the Ubiquitin-Proteasome System via Small Molecules to Degrade "Undruggable" Proteins**

- Discuss innovation and characterization of a cellular platform to identify molecular glue degraders via phenotypic screening
- Learn how coupling said strategy with cutting-edge target-identification approach based on functional genomics and quantitative proteomics led to the identification of novel degraders of cyclin K
- Explore how these and other efforts have culminated in the foundation of Proxygen, a company dedicated to the scalable discovery of molecular glue degraders
- A look at how orthogonal functional genomic approaches enable a quantitative description of resistance mechanisms to targeted protein degraders

Georg Winter, Co-Founder/Principal Investigator, *Proxygen/CeMM*

9:30am Wellness Break

9:35am Presentation: **Pulmonary siRNA Delivery as an Effective Alternative against COVID-19**

- A look at the types of undruggable targets: transcription factors, genetically mutated alleles, viruses in general
- Learn about the universal challenges in the development of small molecule drugs vs. macromolecules (antibodies, nucleic acids), and delivery problems of macromolecules
- Discuss the progress that has been made in this area, with 2 approved RNAi drugs, and the Nobel Prize for CRISPR/Cas

Olivia Merkel, Professor of Drug Delivery, *Ludwig Maximilians Universität München*

10:00am Wellness Break

10:05am Keynote Panel Discussion & Interactive Q&A - **Is Anything Truly Undruggable?**

With pioneering new research and breakthroughs changing the landscape at an incredible rate, it is essential to address the current challenges and opportunities that bind everyone working in the “undruggable” space. Join the leading academics and figureheads in the industry to cover the quintessential points that define this landscape.

- What really defines an “undruggable” target, and what crucial gaps in our scientific understanding are holding us back?
- What are the key universal challenges that affect everyone working in this space?
- What are the main issues in the way of these therapeutics entering into the clinic?
- What progress has been in the last 6 months, and what has this shown us about the future of the industry?
- What can we achieve by bringing the industry together, and what can we expect to take away from this event?

Robert Konrat, Professor of Biomolecular Structural Chemistry, *University of Vienna*

Annemieke Aartsma-Rus, Professor, Translational Genetics, *Leiden University Medical Center*

Georg Winter, Co-Founder/Principal Investigator, *Proxygen/CeMM*

Olivia Merkel, Professor of Drug Delivery, *Ludwig Maximilians Universität München*

10:45am Morning Extended Wellness Break

Catch-up on emails, let the dog out, brew some more coffee... use this time to take care of regular day-to-day life.

Bioavailability, Drug Delivery and the Route to Clinic

11:25am Presentation - **Nucleic Acid Therapeutics: Expanding the Target Space Beyond Rare Diseases**

- Hear about the promise of oligonucleotide based therapeutics beyond rare diseases and the AstraZeneca experience
- Discussion will focus around advances in chemistry and delivery systems to enhance the drug-like properties of oligonucleotides and expansion of target space.

- A look at the future opportunities and challenges in oligonucleotide based therapeutics

Shalini Andersson, Chief Scientist New Therapeutic Modalities and Head of Oligonucleotide Discovery, *AstraZeneca*

11:50am Wellness Break

11:55am Presentation: **Harnessing The Power of Protein Aggregation with Pept-Ins**

- Hear how Aelin Therapeutics are using synthetic peptides to disturb the natural three-dimensional folding process of disease-causing proteins to create functional knockouts through aggregation
- Discuss Pept-In's key element, the APR (aggregation prone regions), which not only induce protein aggregation, but also promote cellular uptake allowing Pept-Ins be used against intractable targets, such as transcription factors or intracellular scaffold proteins

Els Beirnaert, CEO, *Aelin Therapeutics*

12:20pm Wellness Break

12:25pm Presentation: **Converting Innovative Bicycles into Transformative Medicines**

- Hear about a novel new class of therapies of bicyclic peptides: synthetic short peptides constrained to form two loops favourable for drugging historically intractable targets
- Learn about the favourable pharmacokinetic characteristics and toxicological profiles that this unique therapeutic class offers, combining the benefits of a biologic and a small molecule
- A look at Bicycle Therapeutics' two programs against oncology targets, Bicycle conjugates and immuno-oncology, and their collaborations focusing outside this bracket, including anti-infectives and neuroscience

Nick Keen, CSO, *Bicycle Therapeutics*

12:50pm-1:50pm

Lunch

Finally, a lunch at a conference that is completely your choice! Use this extended break to have your regular lunch, spend time with your family and give your brain a break. Recharge for the afternoon session.

Day 1 of The Undruggable Leaders Forum Europe includes an extended break in the middle to give you ample time for lunch and another chance to catch-up on the day job. Or if you have time, you can use this break for 1-2-1 meetings or open networking.

1:50pm Fireside Chat - **Fireside Chat with Darryl McConnell, Boehringer Ingelheim**

- How are Boehringer Ingelheim pushing the boundaries to drug cancer's big 4?
- Which areas in the 'undruggable' space are currently seeing the most growth within BI?
- What does the future hold for BI and for the 'undruggable' industry?

Darryl McConnell, SVP & Research Site Head, Austria, *Boehringer Ingelheim*

2:30pm Wellness Break

Crucial Case Studies

2:35pm Presentation - **Cellular Mechanistic Approaches to Study Undruggable Proteins**

- Overcoming the challenges of undruggables for targeted protein degradation using HaloPROTAC3 paired with HaloTag CRISPR
- Kinetic degradation analysis and target loss phenotype studies
- Exploration of cellular mechanisms in the Ras:Raf pathway

Elizabeth Caine, R&D Scientist, *Promega Corporation*



3:00pm Wellness Break

3:05pm Presentation: **Optimising Antisense Technology For Multiple Routes of Administration**

- Discuss Ionis Pharmaceuticals 40 strong pipeline of first-in-class or best-in-class medicines against a number of diseases, including cancer, cardiovascular, neurological and infectious diseases
- A look at LICA (Ligand-Conjugated Antisense) Technology, developed in-house which allows antisense drugs to be delivered specifically to certain cell types, allowing less frequent and smaller doses
- Understand how Ionis' single-stranded antisense therapies are efficacious through nearly all routes of administration, and are able to reach all diseased parts of the body whilst minimising off-target effects for more precise targeting and a wider variety of targetable diseases

Punit Seth, VP, Medicinal Chemistry, *Ionis Pharmaceuticals*

3:30pm Wellness Break

3:35pm Presentation - **Accelerating MonoDAC™ and BiDAC™ Degradation Design**

- Explore C4 Therapeutics' pioneering TORPEDO™ platform, which uses proprietary assays and quantitative data to accelerate drug design
- Learn how C4 are using this platform to develop both MonoDAC™ and BiDAC™ degraders against a number of oncology targets in-house
- Discuss the potential applications of this platform, and how, through a number of collaborations including Roche and Biogen, it is already being applied to new disease states

Stewart Fisher, CSO, *C4 Therapeutics*

4:00pm Wellness Break

4:05pm Presentation - **Novel Approach for the Discovery of Small Molecules that Selectively Control mRNA Translation as a New Strategy Against Undruggable Proteins**

- Discuss targeting mRNA translation in a tissue-selective and disease-specific manner with small molecules
- Explore novel target space of mRNA biology: from transcription to translation
- A look at Anima's active and selective mRNA translation modulators across multiple therapeutic areas including Fibrosis, Oncology, Infectious Diseases, and Neuroscience

Yochi Slonim, CEO, *Anima Biotech*
Iris Alroy, VP of R&D, *Anima Biotech*

4:30pm Wellness Break

4:35pm Presentation - **Unlocking Druggability with Large-Scale Molecular Dynamics**

- Explore protein dynamics and its implication for druggability
- A look at how long time scale MD simulation can uncover novel druggable opportunities
- Discuss a case study for BCL family protein

Haotian Li, CTO, *Redesign Science*

Redesign
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5:00pm Wellness Break

5:05pm Strategic Panel & Interactive Q&A: **From Start-Ups to Navigating A Pandemic: C-Suite Table**

If working in a cutting-edge industry focused on the most intractable targets wasn't challenging enough, the last 12 months have brought some of the most disruptive changes to healthcare in history. Join us for a deep-dive chat with 4 of the biggest names in the business as we cover everything from building a biotech from the ground up, to keeping it afloat through a pandemic.

- Why did you choose your target/diseases, and what is the potential of your approach?
- What are the associated challenges associated with your approach and how are you working to overcome these?
- What has been the biggest challenge you've faced in the last 12 months?
- Has there been any changes to support/funding/collaborations, and if so how have you dealt with this?
- What advice would you give to younger biotech companies?

Stewart Fisher, CSO, *C4 Therapeutics*

Els Beirnaert, CEO, *Aelin Therapeutics*

Nigel Crockett, CBO, *Bicycle Therapeutics*

Jennifer Petter, Founder & CSO, *Arrakis Therapeutics*

Nicola Thompson, CEO, *Amphista Therapeutics*

Haotian Li, CTO, *Redesign Science*

6:00pm **Close of Day 1**

Day 2: Wednesday, April 14th, 2021

8:00am **Conference Platform Opens**

9:00am **Chair's Opening Remarks**

Mads Aaboe Jensen, External Innovation Officer, RNA Molecule Research, *Roche*

Discovery and Screening

9:05am Keynote Presentation - **Drug Discovery of Protein Degradation and PPI Modulation**

- A look at the last decade of pioneering research that has led to the arrival of a whole new modality of chemical intervention: “tailored” bifunctional molecules for targeted protein degradation
- Hear how the Ciulli laboratory are using a combination of organic and medicinal chemistry, computational tools, structural biology and biophysics to design these molecules and reveal their structural and dynamic interactions
- Explore how these techniques are being utilised to design and develop PROTACs for cancer and other disease states, and for novel approaches to small-molecule inducible degron technologies

Alessio Ciulli, Professor/Founder & Director, *University of Dundee/Amphista Therapeutics*

9:30am Wellness Break

9:35am Presentation - **Discover Novel Molecules with More Efficient Exploration of Chemical Space**

- Explore common data challenges and considerations for improved prediction outcomes
- Discuss a case study: Bayer and CAS collaboration shows impact of high-quality data on prediction of reaction outcomes
- A look at the Custom Services for scientific data, analytics and insights to transform R&D and accelerate discovery

Yugal Sharma, Senior Director, Services, *CAS Services*



10:00am Wellness Break

10:05am Presentation - **Drugging the undruggable with PROTEINi**

- Discuss how protein interference (PROTEINi) is a powerful and unique approach to expanding the druggable space
- Look at how PhoreMost are able to discover novel and unprecedented therapeutic drug targets and simultaneously uncover the means to adequately drug those targets
- A look at how this technology has been applied in key disease areas, including in cancer biology, neurodegeneration and for hijacking targeted protein degradation

Benedict Cross, CTO, *PhoreMost*

10:30am Wellness Break

10:35am Presentation - **Designing Precision Medicines using Fragment Based Drug Discovery**

- Astex has pioneered Fragment Based Drug Discovery (FBDD) and is extending the technology with CryoEM.
- A look at two launched drugs for cancer patients, Kiqali and Balversa.

- Hear about Tolinapant (Phase 2 clinic), an example of FBDD for a protein-protein interaction target, IAP.

David Rees, CSO, *Astex Pharmaceuticals UK*

11:00am Morning Extended Wellness Break

Catch-up on emails, let the dog out, brew some more coffee... use this time to take care of regular day-to-day life.

Rising Stars: Who's Breaking Through?

11:15am Presentation: **A New Approach to Targeted Protein Degradation**

- Hear how Amphista are using the groundbreaking research from Professor Alessio Ciulli's laboratory to build a non-traditional approach to targeted protein degradation
- See how Amphista Therapeutics plan to use this technology to develop an internal pipeline of therapeutics against cancer and other diseases of high unmet need
- A look at the initial preclinical data and next steps for Amphista Therapeutics

Ian Churcher, CSO, *Amphista Therapeutics*

11:35am Wellness Break

11:40am Presentation: **Getting to the clinic with a First-In-Class Direct Myc Inhibitor**

- Learn about Peptomyc's approach against the highly validated "undruggable" oncoprotein Myc: inhibition through cell-penetrating peptides (CPPs)
- A look at the preclinical validation data for the Omomyc mini-protein in the treatment of multiple types of cancer"

- Hear about the ongoing clinical trial Phase I/II studies assessing safety and efficacy of the Omomyc mini-protein.

Laura Soucek, Founder & CEO/ICREA Research Professor, *Peptomyc/Vall d'Hebron Institute of Oncology (VHIO)*

12:00pm Wellness Break

12:05pm Presentation - **Discovery of Small Molecule Drugs Targeting the Biogenesis of microRNA-15 for the Treatment of Systemic Lupus Erythematosus**

- Explore how Saverna Therapeutics have integrated fragment-based screening (FBS) by Nuclear Magnetic Resonance (NMR), in-silico machine learning and cellular assays for to identify ligands for the modulation of RNAs
- Hear about Saverna's workflow RiboSar for RNA targets, which utilises more than 50 machine learning models to predict a hit-to-lead project success and more
- Discover Saverna's mission to treat lupus through inhibition of miR-155 maturation using low molecular weight compounds

Kamal Azzoui, CEO & Co-Founder, *Saverna Therapeutics*

12:25pm Wellness Break

Future Perspectives

12:35pm Panel Discussion & Interactive Q&A - **Examining the Future Portfolio: What's Next?**

Join forward-thinking leaders in the space to examine what the future "undruggable" portfolio will look like, how the last 12 months have affected future investment and collaboration in the industry, and a look at where the industry could potentially be in the next year.

- Which targets, approaches or technologies have the greatest potential for therapeutic impact?
- How has pharma's appetite for risk changed in the wake of COVID-19, and how is this change affecting collaborations against undruggable targets?

- Where do we expect to see more pharma-biotech partnerships being established in the near future?
- What will pharma's future portfolio look like in a more risk-averse world - more or less oncology-focused?
- Which parts of the industry will see the most growth in the next 12 months?

Ian Churcher, CSO, *Amphista Therapeutics*

Andrew Benowitz, Senior Director and Protein Degradation Group Head, *GSK*

Philipp Hoffman, Executive Director, Head Business Development & Licensing, *Daiichi Sankyo Europe GmbH*

Jamie Kasuboski, Venture Investor, *OMX Ventures*

Danette Daniels, R&D Group Leader, *Promega Corporation*

1:25pm **Close of Day 2**