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November 13-15 | Boston, MA

www.complement-therapeutics.com

3rd Annual

Complement-based Drug Development Summit 2019

Optimize Novel Pathway Targeting, Assay Validation & Clinical Translation to Drive Complement Inhibition Development

Expert Speakers Include:

Steve Zelenkofske
CMO
Achillion Pharmaceuticals

Lukas Scheibler
CIO
Apellis Pharmaceuticals

Niels Riedemann
CEO
InflaRx N.V.

Muneto Mogi
Executive Director, Global Discovery Chemistry – Global Discovery Chemistry
Novartis

Barbara Klughammer
Senior Principal Scientist
Roche

Peter Rutherford
Global Medical Lead, Orphan Renal Diseases
Vifor Pharma

Partners:

Machaon Diagnostics
Coagulation, platelets, rare disease and genetics

KYPHA
Making sense of inflammation

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Welcome to the 3rd Annual Complement-based Drug Development Summit

Investment in the complement therapeutics field is exploding fuelled by an increased fundamental understanding.

The next few years are pivotal. As a community we await key trial read-outs that could impact the industry hugely. Although there remain challenges to be faced, there is a growing sense of excitement.

The **Complement-based Drug Development Summit** is the **only industry dedicated meeting** that brings you the most up to date clinical and commercial developments utilizing complement inhibitors in rare and common disease indications.

You will gain key insight on overcoming end to end challenges from preclinical modelling to streamlining process development. This event will enable you to understand the fundamental role of complement in specialized cells to manipulate the complement cascade to treat common and rare diseases.

Join experts from **InflaRx**, **Apellis**, **Novartis**, **Roche**, **Genentech** and **Achillion** to discuss targeting the classical, lectin and alternative pathways and creating robust testing and assay measures for patient selection and response. With over 150 of the industry's pioneers we will gather to advance to the clinic novel complement based drugs beyond C5.

Hear what previous attendees have to say

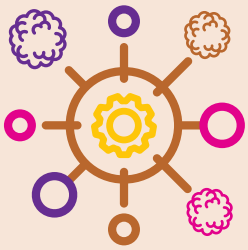
"Bringing together high class academic research and top-notch research from drug development companies within the complement therapeutic space is a new format which brings surprisingly new insights, collaborations and motivation. In a very short time, this has become a small but must-go-to meeting in the complement drug development space. Congratulations to the organizers! Much looking forward to the next meeting"

Niels Riedemann, CEO, InflaRx N.V.

Your Checklist to Developing Complement Therapeutics

Discover the Importance of Gathering Real World Evidence for Development and Launch of New Complement Therapeutics

Presenting patient, HCPs, payers and regulator's interest in real world data and how they must be addressed in complement therapeutics with **Peter Rutherford** of **Vifor Pharma**



Modulate the Alternative Pathway with a Diverse Pipeline of Targets and Modalities

Discover research vignettes covering efforts to target various factors within the complement cascade with **Novartis** experts

Hear Clinical Trial Experience to Accelerate your Complement Therapeutics Through the Clinic

Identify bottlenecks, pressure points and opportunities in clinical trials with **Niels Riedemann** of **InflaRx N.V** and **Lukas Scheibler** of **Apellis Pharmaceuticals**

Revealing How to Create Translatable Assays to Measure the Functional Activity of Complement Therapeutics

Explore the need for standardized assays to minimize variation between labs and accurately determine clear and reliable clinical comparisons

Improving Translational Biomarkers to Enhance Understanding of the Complement System

Identify biomarkers that are driving disease and indicating complement activation to allow accurate measurements of efficacy and endpoints with **Kypha**

YOUR EXPERT SPEAKERS

Francisco Fernández
CEO & Co-founder
Abvance

Steve Zelenkofske
CMO
Achillion Pharmaceuticals

Lukas Scheibler
CIO
Apellis Pharmaceuticals

Jesse Hanson
Research Scientist
Genentech

Janes Hughes
VP of Translational Research
Gyroscope Therapeutics

Rajendra Kumar-Singh
Scientific Founder
Hemera Biosciences

Giulio Pasinetti
Professor
Icahn School of Medicine at Mount Sinai

Niels Riedemann
CEO
InflaRx N.V.

Martin Schmidt
CSO
Kypha

Mike Ero
CEO
Machaon Diagnostics

Eric Furfine
Head of Ophthalmology
Mosaic Biosciences

Carlos Nogueras-Ortiz
Post-Doctoral Researcher
National Institute of Aging

Thomas Smith
Senior Research Investigator, Lead Discovery
Novartis

Christopher Adams
Senior Investigator, Global Discovery Chemistry
Novartis

Muneto Mogi
Executive Director, Global Discovery Chemistry
Novartis

Simon Read
CSO
Ra Pharmaceuticals

Barbara Klughammer
Senior Principal Scientist
Roche

Giles Campion
Head of R&D and CMO
Silence Therapeutics

Peter Rutherford
Global Medical Lead, Orphan Renal Diseases
Vifor Pharma

Karthik Viswanathan
Senior Director
Visterra

Robert Montgomery
Director
NYU Langone Transplant Institute

WORKSHOP DAY WEDNESDAY, NOVEMBER 13

Workshop A

9am-12pm

Exploring the Use of Complement Inhibitors in Transplantation

The classical complement pathway is often activated within the first 9 weeks of transplantation due to early acute antibody-mediated rejection (AMR) being triggered by recipient donor-specific antibodies (DSAs) binding to donor human leukocyte antigens (HLAs). It has been shown that blocking the cleavage of C5 (thus preventing terminal complement activation) using Eculizumab lowers the incidence of acute AMR in highly sensitized recipients of living-donor kidney transplants compared with a historic control group. Just this one example displays the promise of complement inhibitors in transplantation. Understand how to properly design trials to display the efficacy of your complement inhibitor at this workshop.

Attend this workshop to:

- Hear lessons learned from the clinical design of trials in the transplant space
- Discuss data analysis from negative Eculizumab trials
- Discover that changing certain flaws in clinical trial design can lead to stunningly different results
- Debate patient stratification and its importance in clinical trial design

Workshop Leader

Robert Montgomery
Director
NYU Langone Transplant Institute

Robert A. Montgomery, MD, DPhil, FACS, is the Director of the NYU Langone Transplant Institute and a Professor of Surgery. He received his Doctor of Medicine with Honor from the University of Rochester School of Medicine. He received his Doctor of Philosophy from Balliol College, The University of Oxford, England in Molecular Immunology. Montgomery completed his general surgical training, multi-organ transplantation fellowship, and postdoctoral fellowship in Human Molecular Genetics at Johns Hopkins. For over a decade he served as the Chief of Transplant Surgery and the Director of the Comprehensive Transplant Center at Johns Hopkins.

"As an academic researcher, I had a rare and valuable experience to be exposed to the way industry leaders approach some challenging topics, assess various sources of data and take scientific and business decisions. I learned a lot and my perspective has been enriched"

Previous Attendee

Workshop B

1pm – 4pm

The Inflammasomes: Linking Immunological Priming, Neuroinflammation, Complement Activation and the Development of Psychiatric Disorders

We have proposed this workshop based on recent evidence which indicates that the exposure to multiple environmental stressors elicits proportionally stronger neuroinflammatory responses. This workshop will explore the neurochemistry underlying the nexus between immunological priming, the inflammasome complex, the complement system **(CS)**, and microglia initiated neuroinflammation. Recent evidence suggests that immune system-based recognition of the pathogens or their pathogen associated molecular patterns (PAMPs) initiates the pro-inflammatory immune response required for the maintenance of homeostasis.

The overall goal of this workshop is to discuss how inflammasome mediated neuroinflammation contributes to the altered neurochemical signatures found in certain psychiatric disorders such as depression. Pattern recognition receptors (PRRs) including toll-like receptors (TLRs), complement receptors (CRs), and nucleotide-binding oligomerization domain and leucine-rich repeat-containing receptors (NLRs) of inflammasomes regulate both these processes of recognition of pathogens/PAMPs and their clearance have been implicated in novel therapeutic approaches in mental disorders.

Moreover, recent evidence implicates the effect of the microbiome in TLRs mediated responses in the brain, and will be discussed in respect to new evidence of the role of inflammasome and depression. This workshop will include experts in the fields of neuroinflammation, inflammasome activation, complement system, and models of neuroimmunological priming in response to environmental stressors.

Attend this workshop to learn about:

1. The crosstalk between the **CS** and inflammasomes in the generation of pro-inflammatory immune response (eg C1q inactivation of NLRP3 inflammasome and CASP1 activation required for the release of IL-1b).

2. The role of pathogen recognition by the **CS** and their interactions during various immunological processes including autophagy and innate immunity in response to stress induced psychological impairment.

3. The enzymatic reactions that facilitate the transcription and activation of inflammasome complexes such as NLRP3 in the brain and how these complexes activate neuroinflammation, which can lead to synaptic maladaptation observed in depression-like phenotypes.

4. The expanding economic toll of psychiatric disorders that has fueled a demand to better understand the etiology of psychiatric disorders, and to develop new therapeutic approaches using rational drug design (including novel drug influencing the **CS**).

Given the recent focus on both the role inflammasomes play in catalyzing the activation of inflammatory cytokines as well as new therapeutics that target these complexes, now is the time to better understand the biochemical pathways involved in the activation of certain inflammasomes!

Workshop Leader

Giulio Pasinetti
Professor
Icahn School of Medicine at Mount Sinai

Dr. Giulio Maria Pasinetti is the The Saunders Family Chair and Professor of Neurology, Neuroscience, and Geriatrics and Adult Development at the Ichan School of Medicine at Mount Sinai (ISMMS) in NY. He also serves as the Director of Basic and Biomedical Research in the Center for Geriatic Research and Training at the Bronx Veterans Affairs Medical Center. He is also the Director of the Center for Molecular Integrative Neuroresilience at the ISMMS. The Center's goal is to understand the mechanisms of action through which novel therapeutics act against stressful events and to clarify the role of innate immunity in the gut/microbiome-brain axis at the genomic level in the promotion of cognitive and psychological resilience across the lifespan.

"This was a well-balanced mix of speakers representing early and late drug development efforts and approaches that have worked and ones that have failed, we often only hear the success stories, but one can learn maybe even more from the failures"
Ablynx

CONFERENCE DAY ONE
THURSDAY NOVEMBER 14, 2019

8.15 **Coffee & Registration**

9.15 **Chair's Opening Remarks**

Novel Approaches to Understand Complement in Physiology and Disease

Jesse Hanson
Research Scientist
Genentech

9.30 **Classical Pathway Activation in Alzheimer's Disease (AD)**

- Expression of the classical pathway is induced in glial cells in both amyloidosis and tauopathy mouse models
- Knockout of C1q or C3 rescues plaque-associated synapse loss in amyloidosis mice, and ameliorates brain atrophy in tauopathy mice
- C1q and C3 are elevated at synapses in AD patient brains
- Elevated levels and activation of C4 and C3 are detected in AD patient cerebrospinal fluid

Thomas Smith
Senior Research Investigator, Lead Discovery
Novartis

Christopher Adams
Senior Investigator, Global Discovery Chemistry
Novartis

10.00 **A Diverse Pipeline of Targets and Modalities: Novartis' Approach to Modulating the Alternative Complement Pathway**

- Research vignettes covering efforts to target Factor B (LMW), Factor D (LMW), Factor P (Ab) and C5 (Ab and LMW)

11.00 **Morning Refreshments and Speed Networking**

Eric Furfine
Head of Ophthalmology
Mosaic Biosciences

12.00 **CB 2782-PEG: A Complement Factor C3-Inactivating Protease and Potential Long-Acting Treatment for Dry AMD**

- CB 2782-PEG is a 68 kDa engineered protease to specifically cleave C3 into inactive fragments. It has a ~500 kDa MW on SEC due to the large hydrodynamic radius
- The increased size of CB 2782-PEG resulted in enhanced ocular half-life in rabbits (3.9 vs 1.9 days for the unmodified CB 2782) and in non-human primates (3.7 vs. 1.7 days)
- Single intravitreal injection of 125 µg CB 2782-PEG achieved complete, rapid and sustained PD inhibition (>99%) of vitreous C3 for at least 28 days in African green monkeys
- The long pharmacodynamic effect is the result of the catalytic mechanism and a distinct advantage over traditional stochiometric binding inhibitors
- CB 2782-PEG has potential for anti-C3 best-in-class efficacy and convenience in geographic atrophy dry AMD with anticipated intravitreal human dosing three or four times a year

Steve Zelenkofske
CMO
Achillion Pharmaceuticals

12.30 **Development of Oral Complement Factor D inhibitors**

- Update Achillion Development Pipeline
- Report Interim Danicopan Phase 2 Data in PNH Patients
- Provide Topline Summary on 2nd Generation of Oral Complement Factor Inhibitor

Mike Ero
CEO
Machaon Diagnostics

1.00 **Update On the Retrospective MAC Plus Study of 1,000 aHUS Genetic Results and Other Laboratory News**

- Talk details to be confirmed

	1.30	**Lunch & Networking**
Giles Campion Head of R&D and CMO **Silence Therapeutics**	2.30	**A Novel siRNA Approach for the Treatment of Complement-mediated Diseases** • Application of siRNAs in complement cascade interference - where in the complement cascade does use of a GalNAc siRNA make sense? • Benefits of a GalNAc/liver specific treatment approach vs. other modalities • Pre-clinical data from a new GalNAc siRNA therapy
Niels Riedemann CEO **InflaRx N.V.**	3.00	**Developing the First in Class Anti-C5a Antibody IFX-1: Lessons Learned from Clinical Trials** • Exact talk details to be confirmed
	3.30	**Afternoon Refreshments & Poster Session**

4.30 Speed Learning: Exploring Complement Therapeutics in Various Indications & Hearing Novel Solutions

Short yet focused sessions allowing for multiple indications and challenges to be addressed in detail. Each table will be hosted by a leader who will give a 5 minute presentation, you then get the opportunity to discuss the topic and question the host for 10 minutes before moving on to your next table.

- Discover complement's involvement in new indications
- Explore novel methods to evaluate complement targeting therapeutics
- Dive into discussion on groundbreaking complement research

Discussing availability and suitability of animal models for evaluation of complement targeting therapeutics

Karthik Viswanathan
Director - Research
Visterra

Astrocyte-derived extracellular vesicles as carriers of complement-mediated neurotoxicity in Alzheimer's disease and traumatic brain injury

Carlos Nogueras-Ortiz
Post-Doctoral Researcher
National Institute of Aging

Differentiating whether fluid phase vs surface alternative pathway complement activation is casual in disease

Muneto Mogi
Executive Director, Global Discovery Chemistry
Novartis

Complement's role in renal disease: pathogenesis, diagnostics, and therapeutics

Francisco Fernández
CEO & Co-founder
Abvance

	6.00	**Chair's Closing Remarks**
	6.05	**End of Day One**

"The Complement-based Drug Development regrouped the majority of the key players of the Pharmaceutical Industry in the complement field. It is priceless to be able to interact with each other at this level of Science"
Shire Pharmaceuticals

CONFERENCE DAY TWO
FRIDAY NOVEMBER 15, 2019

	8.00	**Coffee & Registration**
	9.00	**Chair's Opening Remarks**
Simon Read CSO **Ra Pharmaceuticals**	**9.10**	**Development of Zilucoplan for Neuromuscular Diseases** • Benefits of a peptide based approach to complement inhibition in neuromuscular diseases • Development of alternative formulations for once weekly subcutaneous dosing • Novel approaches to clinical development in neuromuscular disease
Janes Hughes VP of Translational Research **Gyroscope Therapeutics**	**9.40**	**Utilising Gene Therapy to Target the Alternative Pathway in Age-Related Macular Degeneration** • Introduction to GT005, lead asset at Gyroscope Therapeutics • Pre-clinical data supporting scientific rationale • FOCUS I/II study in dry AMD
Martin Schmidt CSO **Kypha** **Simon Read** CSO **Ra Pharmaceuticals**  **Karthik Viswanathan** Director - Research **Visterra**	**10.10**	**Panel Discussion: Improving Translational Biomarkers to Understand the Involvement of the Complement System in All Indications** • What current signatures suggest that complement is the primary driver of the disease? • Are there innovative biomarkers that can show complement expression? • How can companies improve therapeutic targeting after identifying if complement is a driver or a by-product of an indication? • Improving drug efficacy assessment by streamlining processes for robust in-house functional assay development
	11.10	**Morning Refreshments & Networking**
Peter Rutherford Global Medical Lead, Orphan Renal Diseases **Vifor Pharma**	**11.40**	**Successful Real World Evidence Gathering and Patients Engagement - Vital for Development and Launch of New Complement Therapeutics** This presentation will explore the vital importance of gathering and gaining insight from real world data in successful complement therapeutics developments. • Describing how real world evidence gathering in rare diseases gives insight on burden of disease and unmet medical need which is critical for trial design and creation of the TPP • Emphasising how patient engagement is important in this process through their role in European Reference Networks and disease registries • Presenting the multi-stakeholder interest in real world data in complement therapeutics – patient, HCPs, payers and regulators – and how they must be addressed
Lukas Scheibler CIO **Apellis Pharmaceuticals**	**12.10**	**Apellis: Clinical Updates with a Focus on APL-2** • An update on the ongoing phase 3 clinical studies with APL-2 in hematology and ophthalmology indications will be provided • A review of Apellis' broader pipeline focusing on C3 modulation will be provided
	12.40	**Lunch**
Rajendra Kumar-Singh Scientific Founder **Hemera Biosciences**	**1.40**	**Targeting of the Membrane Attack Complex by Gene Therapy for the Treatment of Age-related Macular Degeneration- from Hypothesis to Two Phase I Clinical Trials** • The membrane attack complex (MAC) is elevated in age-related macular degeneration • CD59 is an inhibitor of the membrane attack complex • Adenovirus-associated virus gene transfer of CD59 inhibits (MAC) deposition • Early results of two AMD trials will be presented
	2.10	**Chair's Closing Remarks**
	2.15	**End of Day Two**

PARTNER

Tailored Opportunities include:

Speaking Opportunities
position yourself as the thought leader in your space and educate the market

Panel Moderator
curate and drive the conversation alongside leaders of the field

Roundtable Leader
interact with your market audience to understand their needs

Workshop Leader
lead a focused and in-depth discussion with a targeted audience

Exclusive VIP Hosting
build or cement relationships through informal channels

Exhibition Stand
have your flag in the ground and showcase your technology to the onsite community of senior decision makers

Program Partner

Machaon Diagnostics
Coagulation, platelets, rare disease and genetics

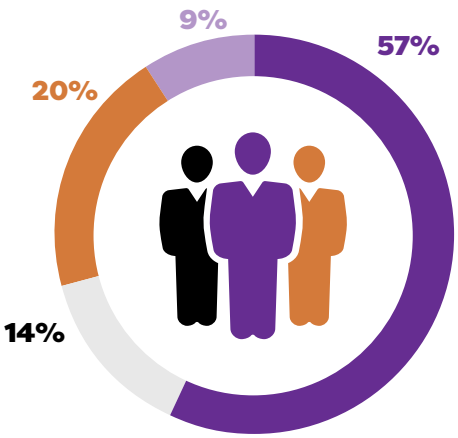
www.machaondiagnostics.com

Hosting Partner

KYPHA
Making sense of inflammation

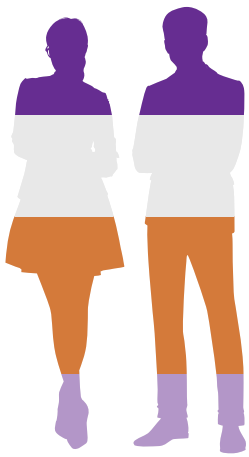
www.kypha.net

LAST YEAR'S AUDIENCE COMPOSITION:



- **Drug Developers**
- Service Providers
- **Healthcare Providers**
- Research Institute

LAST YEAR'S SENIORITY:



- **CXO: 25%**
- **President/VP: 22%**
- **Head/Director: 34%**
- **Scientist/Professor: 19%**

JOIN US

Jonathan Kilby-Phillips
Commercial Director
Tel: +1 617 455 4188
Email: sponsor@hansonwade.com

READY TO REGISTER?

3 EASY WAYS TO BOOK

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Network with the experts and leaders of the Complement community and take this opportunity to make collaborative partnerships for future clinical prospects.

Hear the novel clinical data from trials and apply the lessons learnt to future pipelines to ensure future efficacy.

Learn from the leaders in the space and join the communal discussion finding solutions to limiting manufacture and regulatory issues.

Team Discounts*

- **10% discount –** 3 delegates
- **15% discount –** 4 delegates
- **20% discount –** 5 or more delegates

Please note that discounts are only valid when three or more delegates from one company book and pay at the same time.

Discounts cannot be used in conjunction with any other offer or discount. Only one discount offer may be applied to the current pricing rate.

Contact: register@hansonwade.com

SECURE YOUR PLACE

Standard Pricing	Register & Pay By Friday September 13	Final Registration Prices
Conference + 2 Workshops **(A & B)**	$3597 **(save $900)**	$3997 **(save $500)**
Conference + 1 Workshop **(A or B)**	$3098 **(save $700)**	$3498 **(save $300)**
Conference Only	$2699 **(save $400)**	$3099
Workshops **(Individual)**	$699	

Industry Pricing*	Register & Pay By Friday September 13	Final Registration Prices
Conference + 2 Workshops **(A & B)**	$2897 **(save $800)**	$3297 **(save $400)**
Conference + 1 Workshop **(A or B)**	$2398 **(save $600)**	$2798 **(save $200)**
Conference Only	$1899 **(save $400)**	$2299
Workshops **(Individual)**	$699	

*Pharma and biotech companies currently and publically developing complement therapeutics
Academic discounts available upon request

VENUE

Renaissance Boston Waterfront Hotel
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