Book Before June 7 & Save up to $800

August 20-22, 2019 | Boston, MA

HDD Hemophilia Drug Development

Advancing Next Generation Therapies for Bleeding Disorders

Delivering to Patients Long-Acting, More Effective Factor Replacement Therapies & Curative Gene Therapies

Expert Speakers Include:

Howard Levy
Chief Medical Officer
Catalyst Biosciences

Sander Van Deventer
Chief Scientific Officer
uniQure

Dean Falb
Chief Scientific Officer
LogicBio

Robert Peters
Rare Blood Disorders Therapeutic Area Head, Research
Sanofi Genzyme

Dawn Rotellini
Senior Vice President, Program Development
National Hemophilia Foundation

Didier Rouy
Senior Director of Clinical Sciences
Sangamo Therapeutics

Partners:

athn™ american thrombosis & hemostasis network

Machaon Diagnostics
specialized coagulation testing

CATALYST BIOSCIENCES

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www.hemophilia-drug-development.com

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Overcoming Bleeding Disorders with Long-Acting & Curative Therapies

With the recent Hemlibra approval and the gene therapy revolution we are seeing a **race to market for more successful long-acting or curative** hemophilia and bleeding disorder therapies.

The ***2nd Annual Hemophilia Drug Development Summit*** is dedicated to **translating more effective therapies, bringing the right treatments to meet the goals of every patient**. From subcutaneous therapies to gene therapies these all have the aim of improving patients' quality of life.

This event brings together over 100 of the world's **leading** experts and drug developers. They will discuss the development and **impact of non-factor products**, **patient advocacy and engagement strategies**, **improve testing methods and assays** for better measures of response and diagnosis, and **the payer and reimbursement strategies** to **ensure market access.**

Join the likes of **Sanofi, Novo Nordisk, CSL Behring, Expression Therapeutics, Catalyst Biosciences** and **Bayer**, to name a few, as we look to the innovation driving the field towards advancing the next generation of bleeding disorder therapies.

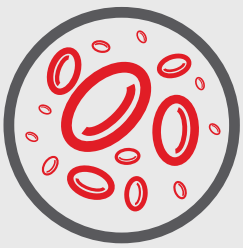
What previous attendees have said about our meetings

Outstanding conference!
Past attendee from the National Hemophilia Foundation

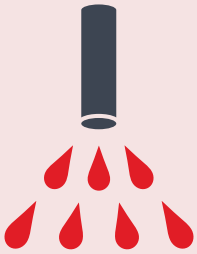
Great meeting flow. The opportunity to network was very beneficial
Past attendee from CSL Behring

Navigate the Clinical to Commercial Pathways for Bleeding Disorder Therapies

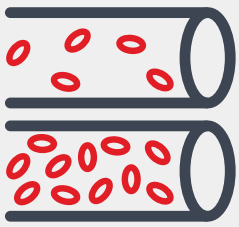
Address R&D, patient engagement and payer challenges of gene therapy with insights from the likes of **uniQure** and **Spark Therapeutics**



Advance novel subcutaneous prophylaxis for inhibitor patients, learn from **Catalyst Biosciences, Sanofi** and **Novo Nordisk**



Optimize clinical development strategies in dedicated roundtable discussions on **patient recruitment** and **defining suitable endpoints**



Bridge the knowledge gap in the diagnosis and treatment of **Von Willebrand Disease**, with **CSL Behring** and the **National Hemophilia Foundation**

Understand the needs of patients and insight from treaters, with expert knowledge from **Bayer** and the **Boston Hemophilia Center**

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Your Expert Speakers

Crystal Watson
President & Chief Executive Officer
American Thrombosis & Hemostasis Network

Olubunmi Afonja
Director, Medical Affairs, Hematology
Bayer

Aric Parnes
Associate Director
Boston Hemophilia Center

Howard Levy
Chief Medical Officer
Catalyst Biosciences

Grant Blouse
Vice President, Translational Research
Catalyst Biosciences

Henry Mead
Director, Medical Communication & Education Strategy Hematology Medical Therapeutic Area Coagulation
CSL Behring

H. Trent Spencer
President
Expression Therapeutics

Dean Falb
Chief Scientific Officer
LogicBio

Michael Ero
Chief Executive Officer & President
Machaon Diagnostics

Brendan Hayes
Director of External Affairs
National Hemophilia Foundation

Michelle Rice
Senior Vice President, External Affairs
National Hemophilia Foundation

Dawn Rotellini
Senior Vice President, Program Development
National Hemophilia Foundation

Jesper Haaning
Vice President, Global Research Project & Biopharm Management
Novo Nordisk

Robert Peters
Rare Blood Disorders Therapeutic Area Head, Research
Sanofi Genzyme

Shauna Andersson
Clinical Research Director
Sanofi Genzyme

Nisha Jain
Global Head, Life Cycle Management, Rare Blood Disorders
Sanofi Genzyme

Didier Rouy
Senior Director of Clinical Sciences
Sangamo Therapeutics

Tessa Field
Patient Advocacy Lead
Spark Therapeutics

Sander Van Deventer
Chief Scientific Officer
uniQure

Pre-Conference Focus Day
Tuesday, August 20 2019

Workshop A 9am - 12pm

Understanding Immunogenicity in Next Generation Factor Replacement Prophylaxis

Novel therapeutic strategies have been able to gradually improve the standard of care of hemophilia treatments. The major barrier to the success of this is the development of inhibitors in the patients. Attending this workshop will help you understand how immunogenicity risk assessment studies and real world data can help you to understand the why certain factor replacement products are more immunogenic.

Attendees will discuss in detail:

- Immunogenicity in extended half-life products, non factor replacement products and gene therapy
- Why antibodies form in response to the different treatments
- Strategies in eliminating immunogenic response and re-instate immune tolerance

Workshop Leader

Grant Blouse
Vice President, Translational Research
Catalyst Biosciences

Nisha Jain
Global Head, Life Cycle Management, Rare Blood Disorders
Sanofi Genzyme

Workshop B 1pm - 4pm

Improving Patient Access to Gene Therapy Through Novel Payer and Reimbursement Strategies

With the iminent approval of gene therapy, existing payment approaches need to be reassessed to ensure better patient access and better value for across the system. Engaging with payers and providers to evaluate and innovate more value-based payments will be key to the success of gene therapy in the real world. The National Hemophilia Foundation is actively working with patients, payers and providers to improve management of the costs of hemophilia treatments. In this workshop, learn about the work of the Comprehensive Care Sustainability Collaborative in improving payer and provider engagement.

Attendees will discuss:

- Understanding what payers are looking for to evidence the curative or long-lasting benefit of gene therapy
- Alternative payment strategies to address affordability of gene therapy
- Challenges around implementation of new payment models

Workshop Leader

Michelle Rice
Senior Vice President, External Affairs
National Hemophilia Foundation

Brendan Hayes
Director of External Affairs
National Hemophilia Foundation

Conference Day One
Wednesday, August 21 2019

8.00 **Registration & Coffee**

8.45 **Chair's Opening Remarks**

Evaluating the Impact of Non-factor Products in a Changing Hemophilia Treatment Paradigm

Aric Parnes
Associate Director
Boston Hemophilia Center

9.00 **Assessing the Impact of New Therapies on Hemophilia Patients**

- Understanding patient preferences in the HAVEN trials
- Evaluating surgical outcomes within emicizumab trials
- Clarifying the role of new therapies in inhibitor patients

Jesper Haaning
Vice President, Global Research Project & Biopharm Management
Novo Nordisk

9.30 **Clinical Development of Concizumab, a Monoclonal Antibody for Subcutaneous Prophylaxis for all Hemophilia Patients**

- Assessment and discussion of Concizumab phase II data
- Treatment that improve clinical outcomes and improve quality of life
- Future development plans

Crystal Watson
President & Chief Executive Officer
American Thrombosis & Hemostasis Network

10.00 **American Thrombosis and Hemostasis Network - A National Collaborative**

ATHN proudly collaborates with researchers to provide a variety of opportunities through our national network of hemophilia treatment centers. Learn more about:

- ATHN core services
- Partnering with ATHN to conduct research
- Current and future projects

10.30 **Speed Networking & Morning Refreshments**

Michael Ero
Chief Executive Officer & President
Machaon Diagnostics

11.30 **Application of State-of-the-art Laboratory Techniques to an Ever-changing Hemophilia Drug Landscape**

- New tests for new drugs
- Appropriate test selection when evaluating new hemophilia therapies
- Linkage between clinical trial outcomes and laboratory work-ups

Shauna Andersson
Clinical Research Director
Sanofi Genzyme

12.00 **Fitusiran, An RNAi Therapeutic Targeting Antithrombin to Restore Hemostatic Balance in People with Hemophilia**

- Fitusiran is a monthly subcutaneous therapy that can treat Hemophilia A, B with and without inhibitors
- Consistent level of protection without peaks or troughs
- Rebalance the hemostatic system

Advancing Novel Prophylaxis that Improves Quality of Life for Patients

Howard Levy
Chief Medical Officer
Catalyst Biosciences

12.30 **A Tale of Two Subcutaneous Coagulation Factors**

- High potency clotting factors are required for subcutaneous (SQ) delivery
- Pharmacokinetics (PK) of SQ delivery results in prolonged half-life and summation of blood levels
- SQ coagulation factors have very different PK: Cmax, Tmax, half-life, mean residence time, AUC and distribution across compartments
- Activity levels are more sustained with SQ injection than intravenous (IV) infusion
- Prophylaxis can be achieved with SQ delivery and is particularly convenient for pediatric patients

1.00 Lunch & Networking

Robert Peters
Rare Blood Disorders Therapeutics Area Head, Research
Sanofi Genzyme

2.00 Research and Development of Next Generation Prophylaxis

- BIVV001 phase 1/2a single dose and repeat dose studies
- Improving the treatment paradigm for patients and physicians

Robert Peters
Rare Blood Disorders Therapeutics Area Head, Research
Sanofi Genzyme

Jesper Haaning
Vice President, Global Research Project & Biopharm Management
Novo Nordisk

Aric Parnes
Associate Director
Boston Hemophilia Center

2.30 Panel Discussion: Improving Current Standard of Care

- Moving towards personalised treatment options
- What are the roles of each therapy at what stage
- Is there a need for an extended factor half-life product?
- Defining protection and goal for treatment
- New product treatment paradigm
- How are novel therapies impacting the market

3.30 Afternoon Refreshments & Scientific Poster Session

4.00 Roundtable Discussion

Be a part of this practical and highly interactive breakout roundtables where attendees can crowd-source solutions and share opinions around pre-assigned topic areas. This is a valuable chance for you to unite with your peers around hot topics and debate best practice.

1. Patient Recruitment Strategies in Clinical Trials in a Space Saturated with Novel Therapies for Faster Approval

- Patient centric approaches to clinical trials
- How to motivate and educate patients for sufficient patient population
- Strategies for improving patient retention

2. Defining Suitable Endpoints to Ensure Consistency in Products Being Evaluated

- Impact on patient lives and what is considered a successful treatment
- How we differentiate modalities and evaluate new drugs coming to the market
- Maximising the use of patient report outcomes for monitoring and assessment

3. Improving Diagnosis and treatment of Von Willebrand

- Genetic testing for Von Willebrand to determine mutation and carrier status
- Underdiagnoses of Von Willebrand Disease and percentage currently require a factor treatment
- Improving awareness and taking concerns seriously

5.00 Chair's Closing Remarks

5.15 End of Day 1

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Conference Day Two
Thursday, August 22 2019

	8.00	**Coffee & Registration**
	8.45	**Chair's Opening Remarks**
		Advancing Gene Therapies into the Clinic
Sander Van Deventer Chief Scientific Officer **uniQure**	**9.00**	**Significance of Neutralizing Antibodies in Gene Therapy in Hemophilia** • Can a titer that impedes transduction be defined • Impact of vector on neutralizing antibodies • Neutralizing antibodies and clinical trials
H. Trent Spencer President **Expression Therapeutics**	**9.30**	**Emerging Roles of Transgene Mutations in Gene Therapy Products for Hemophilia** • Protein engineering strategies to increase protein expression and specific activity of Factor VIII and Factor IX proteins • Immunogenicity of variant proteins and clinical concerns • How various Factor VIII mutations are moving into clinical applications
Dean Falb Chief Scientific Officer **LogicBio**	**10.00**	**A Stable & Durable Treatment for Pediatric Patients with Hemophilia** • Canonical gene therapy cannot be used to treat pediatric patients due to episomal DNA dilution and lack of therapeutic durability • GeneRide provides a safe and durable platform for treating pediatric hemophilia patients through insertion of therapeutic transgenes into the chromosome • Preclinical data in Hemophilia B mouse models demonstrates the safety and efficacy of this therapeutic approach
	10.30	**Morning Refreshment & Networking**
Didier Rouy Senior Director of Clinical Sciences **Sangamo Therapeutics**	**11.00**	**Preliminary Data for DB-525 for the Treatment of Severe Hemophilia A** • Study design • Review of safety • Review of clinical efficacy
		Maximizing Patient Engagement with Novel Technology
Tessa Field Patient Advocacy lead **Spark Therapeutics**	**11.30**	**Improving Patient Education to Build an Informed, Engaged and Empowered Patient Population** • Understanding the questions, concerns, and knowledge gaps of the patient community • Bridging communication between industry and the patient community • Improving patient assess to balanced scientific information
	12.00	**Lunch & Networking**

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Addressing Medical Considerations to Improve Standard of Care

Nisha Jain
Global Head, Life Cycle Management, Rare Blood Disorders
Sanofi Genzyme

1.00 Data from a Prospective Study using FVIIIFc for ITI in Hemophilia Patients with Inhibitors
- Interim data from an ongoing studying
- Predicting responders and non-responders
- Role of high dose in tolerization success

Henry Mead
Director, Medical Communication & Education Strategy Hematology Medical Therapeutic Area Coagulation
CSL Behring

1.30 Women's Health in Von Willebrand Disease
- Relationship between heavy menstrual bleeding and Von Willebrand Disease
- Underdiagnoses of Von Willebrand Disease and percentage currently require a factor treatment
- Characterising disparities among different populations and age demographics
- Genetic profiles and bleeding assessment tools

2.00 Afternoon Refreshments & Networking

Dawn Rotellini
Senior Vice President, Program Development
National Hemophilia Foundation

3.00 Patient Advocacy and Improving Diagnosis of Von Willebrand Disease
- Devising New guidelines for the diagnosis and treatment of Von Willebrand Disease
- Genetic testing for Von Willebrand to determine mutation and carrier status
- Under-diagnoses of Von Willebrand Disease and percentage currently require a factor treatment
- Improving awareness and taking concerns seriously

Olubunmi Afonja
Medical Director
Bayer

3.30 Challenges and Considerations in Medical Affairs for Novel Hemophilia Drug Development
- Understanding the needs for patients
- Representativeness for minority groups
- Pharmacoeconomic considerations

4.00 Chair's Closing Remarks

4.15 Close of the HDD Summit

"I thought the conference exceeded my expectations"
2018 past attendee from Catalyst Biosciences

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Partners

Expertise Partner

The American Thrombosis and Hemostasis Network (ATHN) is a nonprofit dedicated to improving the lives of people affected by bleeding and clotting disorders. We partner with the medical community, government, and industry to provide a technological infrastructure to secure and manage data, support research, measure outcomes, and transform care. Over 38,500 patients have signed up to share their demographic, clinical, and genetic information as part of the ATHNdataset for research. To date, ATHN has launched nine research projects that rely on this valuable data.

www.athn.org

Expertise Partner

Founded in 2003, Machaon Diagnostics is a preferred laboratory partner to pharmaceutical firms conducting clinical trials in the areas of thrombosis, hemostasis, nephology and rare disease. We offer both CAP/CLIA and GLP testing and regulatory-compliant method validation solutions for submission to the FDA and other regulatory bodies. Machaon Diagnostics has emerged as a leading laboratory for complement gene sequencing and other testing, due to our rapid turnaround and expedited study capabilities.

www.machaondiagnostics.com

Industry Partner

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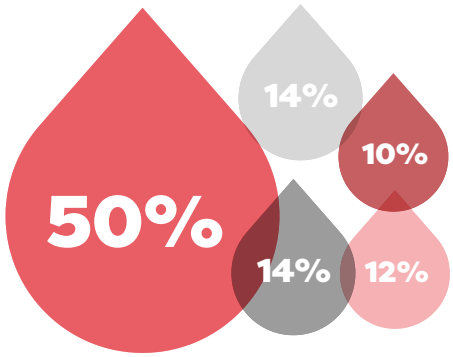
www.catalystbiosciences.com

Why Partner?

The **2nd Hemophilia Drug Development Summit** is the only dedicated meeting focused on overcoming the translational and drug development challenges faced by pharma, biotech and academia looking to clinically develop and commercialize next generation therapies for genetic and acquired bleeding disorders.

Partnering with the **2nd Hemophilia Drug Development Summit** will ensure you capitalize on the market share early. It will enable you to cement your position as an industry leader and support the growing R&D investment that is occurring as researchers strive to pioneer the next wave of safer and more effective therapies that significantly improve patients' lives and health outcomes.

ATTENDEES BY COMPANY TYPE



Drug Developers: 50%

Equipment & Service Providers: 14%

Research Institute: 10%

Healthcare Providers: 14%

Other: 12%

*Audience demographic based on 2018 event data

Partner With Us

Luke O'Neill
Partnerships Director
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Ready to Register?

3 easy ways to book

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Team Discounts
20% discount: 5 or more delegates
15% discount: 4 delegates
10% discount: 3 delegates

REGISTER BEFORE June 7 and SAVE UP TO $800

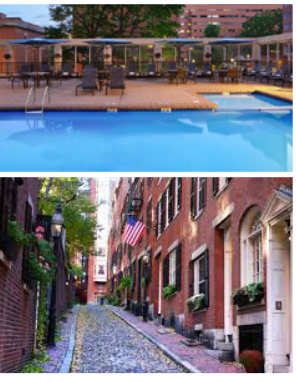
SECURE YOUR PLACE

Drug Developer (Pharma & Biotech)	Register & pay by Friday, June 7	Standard Pricing
Conference + 2 Workshops	$2,697 **(save $600)**	$3,197
Conference + 1 Workshop	$2,198 **(save $500)**	$2,598
Conference Only	$1,699 **(save $400)**	$2,099
Workshops (Each)	$599	

Academic Research Institute & Payers	Register & pay by Friday, June 7	Standard Pricing
Conference + 2 Workshops	$2,197 **(save $500)**	$2,497
Conference + 1 Workshop	$1,698 **(save $400)**	$1,998
Conference Only	$1,199 **(save $300)**	$1,499
Workshops (Each)	$599	

Standard*	Register & pay by Friday, June 7	Standard Pricing
Conference + 2 Workshops	$3,297 **(save $800)**	$3,697
Conference + 1 Workshop	$2,798 **(save $600)**	$3,298
Conference Only	$2,299 **(save $400)**	$2,699
Workshops (Each)	$699	

*If you do not fit either the above pricing you are required to pay this rate



Venue

Wyndham Boston Beacon Hill
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For further information or assistance, please visit www.wyndhambeaconhill.com

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