BOOK EARLY AND SAVE $800

August 14th-16th | Boston

HDD
Hemophilia Drug Development

Advancing Next Generation Therapies for Bleeding Disorders

The Industry Dedicated Summit Focused on Understanding Biological Impacts, Optimizing Clinical Trial Design & Defining Suitable Endpoints for Novel Hemophilia Treatments

Expert Speakers Include:

Robert Peters
Senior Vice President Research
Bioverativ

Henry Mead
Global Medical Director Hematology
CSL Behring

Jesper Haaning
Project Vice President, Global Development
Novo Nordisk

Alison Schecter
Global Program Head, Rare Disease
Sanofi Genzyme

Howard Levy
Chief Medical Officer
Catalyst Biosciences

Sabah Sallah
Vice President, Gene Therapy, Liver Directed Diseases
UniQure

Mark Skinner
President & CEO
Institute for Policy Advancement

Leonard Valentino
Vice President, Hematology
Spark Therapeutics

Chris Haskell
Vice President, Head of the West Coast Innovation Center
Bayer

Tel: +1 617 455 4188 **Mail:** info@hansonwade.com
www.hemophilia-drug-development.com

hansonwade

Overcome Hurdles in the Development & Market Access of Novel Hemophilia Treatments

Fueled by investment and revolutionary therapeutic strategies to **improve current standard of care**, the *Hemophilia Drug Development Summit* will assist you in future-proofing your strategy for translating these most innovative treatments into the clinic.

This summit is your opportunity to immerse yourself in the most innovative **research and development of next generation prophylaxis and gene therapy.**

Have in-depth discussions on **overcoming immunogenicity, preventing inhibitors, optimizing clinical trial design, defining suitable endpoints and exploring reimbursement models** to ultimately ensure success of your therapy.

Join leaders from **Bioverativ, Sanofi, Novo Nordisk, CSL Behring and Spark** as they share their experiences in differentiating their next generation treatments to ensure better quality of life for patients.

What previous attendees have said about our meetings

This was a well-balanced mix of speakers representing early and late drug development efforts, sharing both 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

Past Attendee at the Complement-Based Drug Development Summit: Ablynx NV

Navigate the Development of Revolutionary Treatments from Bench to Bedside

1. Understand the factors that influence patients to adopt novel therapeutics to enhance your research strategy

2. Optimize patient engagement and patient-centric approaches to ensure clinical trial success

3. Gain insight into the scientific rationale and mechanisms in novel methods in dealing with inhibitors to restore the effectiveness of your therapy

4. Learn about works done to minimize joint damage and improve quality of life for patients

5. Explore how different therapies are attractive for pediatric patients for improved patient compliance

6. Evaluate the most accurate physiological correlation for accurate assays to improve safety

7. Learn from recent clinical trials in gene therapy and dissect the variability of results, stability and duration of expression to avoid pitfalls

8. Improve clinical trial design to minimize time and cost

9. Ensure high quality viral vector production while lowering costs

10. Discuss in-depth reimbursement models to ensure effective market access

Tel: +1 617 455 4188 **Mail:** info@hansonwade.com
www.hemophilia-drug-development.com

hansonwade

Your Expert Speakers

Lotta Jansson
Chief Research Offier
Apitope

Chris Haskell
Vice President, Head of the West Coast Innovation Center
Bayer

Robert Peters
Senior Vice President Research
Bioverativ

Howard Levy
Chief Medical Officer
Catalyst Biosciences

Henry Mead
Global Medical Director Hematology
CSL Behring

Michelle Rice
Senior Vice President
External Affairs National Hemophilia Foundation

Mark Skinner
President & CEO
Institute for Policy Advancement

Michael Ero
President and Founder
Machaon Diagnostics

Robert Kotin
Professor
University of Massachusetts Medical School

Christopher Walsh
Director, Hemophilia Program
Mount Sinai School of Medicine

Elizabeth Stoltz
Advancy and Policy Consultant
National Hemophilia Foundation

Jesper Haaning
Project Vice President, Global Development
Novo Nordisk

Irina Matytsina
International Medical Director, Global Development
Novo Nordisk

Valder Arruda
Chair, Gene Therapy and Vaccines Program
University of Pennsylvania

Craig Benson
Clinical Research Director
Sanofi Genzyme

Alison Schecter
Global Program Head, Rare Disease
Sanofi Genzyme

Leonard Valentino
Vice President, Hematology
Spark Therapeutics

Sabah Sallah
Vice President, Gene Therapy, Liver Directed Diseases
UniQure

Daniel Leonard
Director of Global Patient Advocacy
UniQure

"Great conference. The agenda was very focused and stayed within the defined scope"

Past Attendee at the Gene Therapy for Rare Disorders Summit: TrakCel

Conference Day One | Wednesday August 15th, 2018

8.45 Chair's Opening Remarks

Christopher Walsh
Director, Hemophilia Program
Mount Sinai School of Medicine

9.00 Hemophilia: A Model for New Innovations & Competing Technologies in a Rare Disease
- The classic paradigm of protein factor replacement is changing for hemophilia treatment
- Several new 'non-factor' therapies are poised to change hemophilia treatment
- What factors are likely to determine patient acceptance of new technologies?

Mark Skinner
President & CEO
Institute for Policy Advancement

9.30 How to Think about the Future Development of Treatments to Maximize Benefits for Patients
- Bridging the gap between patients, clinicians, regulators and payers
- Improving the consistency of patient reported outcomes
- Educating payers about the value of novel therapies

10.00 Speed Networking
This session is the ideal opportunity to get face-to-face time with many of the brightest minds working in the hemophilia space and establish meaningful business relationships to pursue for the rest of the conference

10.30 Morning Refreshments

Research & Development of Next Generation Prophylaxis

Robert Peters
Senior Vice President Research
Bioverativ

11.00 Developing a New Class of Extended Half Life FVIII Therapies Beyond the VWF Ceiling (BV-EHL FVIII)
- Preclinical and clinical data demonstrate that EHL FVIII molecules that bind VWF reach a maximum threshold
- Using this biology as a guide, molecular engineering of a FVIII fusion protein can overcome the VWF ceiling while still retaining full FVIII activity (rFVIII-VWF-XTEN)
- Multiple types of in vivo and in vitro activity assays are necessary to ensure that potency is correctly assigned
- The implications of modifications to FVIII and FIX must be considered when assessing their biological impact; preclinical examples will be presented including:
  - Ability of VWF-independent FVIII to effectively stop bleeds in vivo, challenging the theory that VWF delivers FVIII to the developing clot
  - Modifications to FIX can alter distribution to the extravascular space, and change the relationship between trough and efficacy

Howard Levy
Chief Medical Officer
Catalyst Biosciences

11.30 Subcutaneous Delivery of Coagulation Factors
- High potency clotting factors are required for subcutaneous (SQ) delivery
- Pharmacokinetics of SQ delivery results in prolonged half-life and summation of blood levels
- Activity levels are more sustained with SQ injection than intravenous (IV) infusion
- Prophylaxis can be achieved with SQ delivery and is particularly attractive for pediatric patients
- SQ injection provides greater convenience than IV infusion

Henry Mead
Global Medical Director Hematology
CSL Behring

12.00 Factor IX Extended Half-Life Therapy: What Have We Learned from the Clinical Trials & Early Use Experience?

- Factor IX replacement therapy has seen significant improvement in half-life extension
- The rate of adoption of FIX EHL concentrates in the Hem B community has been slower than expected
- Early use experience mirrors the registration studies
- Uses in populations beyond the phase three patients will add to the clinical utility of FIX EHLs
- Is gene therapy the true disruptive technology for Hem B therapy?

12.30 Lunch & Networking

Harnessing Novel Non-Factor Replacement Therapies

Jesper Haaning
Project Vice President, Global Development
Novo Nordisk

13.00 Focusing on Antibody Therapies to Identify Opportunities & Challenges

- Mechanistic and PK/PD studies of these therapies
- Addressing the possible long term effects of non-clotting factor treatments
- How to manage breakthrough bleeds

Craig Benson
Clinical Research Director
Sanofi Genzyme

13.30 Restoring Hemostatic Balance in Hemophilia with Fitusiran

- Review of the role of thrombin in hemophilia
- Describing the mechanism of action of Fitusiran, a RNAi targeting Antithrombin
- Overview of the clinical experience of Fitusiran

Improving Clinical Trial Design to Minimize Time & Cost

Irina Matytsina
International Medical Director, Global Development
Novo Nordisk

14.00 Defining Suitable Endpoints in the era of new drugs for Hemophilia

- Clinical relevance and what is considered a successful treatment
- Evaluation of efficacy: is measuring Annual Bleeding Rate (ABR) the only way to do it?
- Utility of patient report outcomes for regulatory approval and market access

Alison Schecter
Global Program Head, Rare Disease
Sanofi Genzyme

Jesper Haaning
Project Vice President, Global Development
Novo Nordisk

Daniel Leonard
Director of Global Patient Advocacy
UniQure

Elizabeth Stoltz
Advancy and Policy Consultant
National Hemophilia Foundation

14.30 Panel discussion: Patient Centric Approaches & Digital Health Solutions to Optimize Patient Engagement

- Patient-centric approaches to treatments
- How to motivate and educate patients for sufficient patient population in clinical trials
- Strategies for improving patient retention
- Implementing and integrating technology to encourage personalized care
- Understanding the regulatory landscape involving technology
- Interrogating the most appropriate wearables, apps, sensors in clinical trial design to improve compliance

15.30 Afternoon Break & Scientific Poster Session

16.15 Roundtable Discussion

1. Developing the Most Accurate Assays for Measuring Therapies to Ensure Best Protection

- Finding the most accurate physiological correlation
- What is the factor equivalent?
- Understanding what is the level of protection during a breakthrough bleed to prevent thrombotic events

2. Understanding Immunogenicity in Factor Products to Eliminate Inhibitor Development

- Mechanism of actions inhibitors
- Proposed strategies for immune suppression and protein modification
- Genetics of inhibitor development

3. In-depth Discussion on Pricing and Reimbursement to Warrant Market Access

- Understanding the reimbursement systems in various countries
- Exploring view points from payers from different healthcare systems

	17.00 Chair's Closing Remarks
	17.15 End of Day 1

"I really enjoyed the broad spectrum of topics that the conference covered and the networking opportunities"

Past Attendee at the Gene Therapy for Rare Disorders Summit: Sanofi

Tel: +1 617 455 4188 Mail: info@hansonwade.com
www.hemophilia-drug-development.com

Conference Day Two | Thursday 16th August, 2018

8.45 Chair's Opening Remarks

Advancing Gene Therapy into the Clinic

Chris Haskell
Vice President, Head of the West Coast Innovation Center
Bayer

9.00 Expectations from Gene Therapy to Establish Advantages and Current Challenges
- Evaluating the advantages and challenges of gene therapy for patients
- What are the long term impacts on patients?
- What are the limitations to gene therapy being curative?

Leonard Valentino
Vice President, Hematology
Spark Therapeutics

9.30 Gene Therapy and Managing Joint Damage to Improve Quality of Life for Patients
- Understanding the basic science behind joint damage caused by hemophilia
- Assessing novel biomarkers
- High quality probing tools that are cost effective improve
- Evidence of novel therapies associated with significant in preventing joint deterioration

Valder Arruda
Chair, Gene Therapy and Vaccines Program
University of Pennsylvania

10.00 Gene Therapy for Inhibitors in Hemophilia
- The use of AAV liver gene therapy has the ability to eradicate and induce immune tolerance to the transgene in canine hemophilia models
- Once inhibitor is eradicated, no need to maintain prophylaxis (to prevent anamnestic responses) and the disease phenotype is improved by continuous expression even at low levels
- AAV liver gene therapy is an alternative for patients refractory to immune tolerance induction

10.30 Morning Refreshments & Networking

Sabah Sallah
Vice President, Gene Therapy, Liver Directed Diseases
UniQure

11.00 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

Lotta Jansson
Chief Research Offier
Apitope

11.30 Prevention of FVIII Inhibitor Formation Using Pan DR Binding ATX-F8-117: Preclinical Efficacy in Humanized Transgenic Mice
- Peptide immunotherapy to re-instate immune tolerance
- Apitope properties and suggested mechanism of action
- Apitopes derived from hFVIII prevents inhibitor generation in preclinical models

Leonard Valentino
Vice President, Hematology
Spark Therapeutics

Sabah Sallah
Vice President, Gene Therapy, Liver Directed Diseases
UniQure

Valder Arruda
Chair, Gene Therapy and Vaccines Program
University of Pennsylvania

12.00 Panel Discussion: A Balanced Evaluation of Recent Clinical Trials to Gain Insights from Leaders in the Field
Shared insight from key opinion leaders with a focus on:
- Dissecting the variability of results, stability and duration of expression
- Ascertaining the appropriate dosage
- Lessons learned from past clinical trials

13.00 Lunch & Networking

Manufacturing & Upscaling to Ensure Drug Quality

Robert Kotin
UMass Medical School

14.00 Ensuring High Quality AAV to Gain a Competitive Edge

- Understanding barriers to quality viral vector production
- Methods for preventing or removing impurities
- Relationship between immunogenicity and manufacturing platforms

Pricing & Reimbursement for next Generation Therapies

Elizabeth Stoltz
Advancy and Policy Consultant
National Hemophilia Foundation

14.30 Fresh Look at Payment Models to Ensure Commercial Success & Patients Benefit

- How do we ensure patient access while demonstrating value of next generation therapies
- Novel strategies for financing treatments
- What happens if a patient changes insurers?

Michelle Rice
Senior Vice President
External Affairs
National Hemophilia Foundation

15.00 Improving Patient Outcomes and Clinical Effectiveness through Collaboration - The Comprehensive Care Sustainability Collaborative

- Obstacles and Barriers impeding patient access to care today
  - Communication
  - Understanding of individual stakeholder drivers
- How do we demonstrate cost quality, Cost effectiveness and Optimal patient outcomes?
- How can a program like CCSC impact the health care landscape, specifically with respect to emerging therapies?
  - Predict/Identify emerging coverage trends
  - Patient/Payer/Provider Engagement
  - Build Consensus & Facilitate proposed solutions

15.30 Chair's closing remarks

15.45 Close of Summit

This conference was one of the most organized ones that I have ever attended. I have been in this business for 32 years. I know the difference. The most interesting part was the "speed dating" rotation. Many people are shy and do not initiate a conversation, however, this game forced everyone to communicate. I enjoyed it immensely. Thank you all for this great, productive, informative and fun event

Past Complement-Based Drug Development Summit Attendee: Quidel

Pre-Conference Workshop Day | Tuesday, August 14, 2018

Workshop A

Addressing Immunogenicity in Hemophilia Treatments to Prevent Inhibitors

9:00am – 12:00pm

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 inhibitor in the patients. Attending this workshop will help you understand why immunogenicity arises, how the manufacturing process can affect antibody production and methods for inhibitor eradication and prevention.

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

Lotta Jansson
Chief Research Offier
Apitope

Lotta Jansson is Chief Research Officer at Apitope International NV, Diepenbeek, Belgium. She gained her PhD at Uppsala University in 1994 and continued an academic career at Lund University for the following 5 years. In 2013, she joined Apitope at the position as Research Director from AstraZeneca where she, for the previous 13 years, had leading scientific positions within areas of autoimmunity and inflammation, inflammatory bowel disease (IBD) and neuroscience at research sites located in Sweden and United Kingdom. Dr Jansson has more than 40 scientific peer reviewed publications and patents.

Workshop B

Optimizing Clinical Trial Design & Overcoming Regulatory Hurdles to Advance Your Pipeline

1:00pm – 4:00pm

As clinical investigations continue to produce promising results, challenges in patient recruitment, determination of endpoint and understanding the regulatory landscape could significantly slow the progression of your pipeline. This workshop is intended to explore the best strategies for clinical trial design and optimizing cost effective late-phase operation.

Attendees will discuss in detail:

- What regulators are looking for in stages of development
- Strategies to optimizing clinical trial design and shortening the time to trial completion
- Optimizing data collection and importance of the site training to ensure high quality data
- Case studies to guide through regulatory strategy and the development process

Irina Matytsina
International Medical Director, Global Development
Novo Nordisk

Irina Matytsina is an International Medical Director in Medical and Science Biopharm, Global Development at Novo Nordisk in Copenhagen. She is responsible for the medical and scientific aspects of phase 3 and 4 clinical trials for hemophilia therapeutics. Irina joined Novo Nordisk in 2010 after having held positions in drug development in several Danish pharmaceutical companies. She has previously worked in clinical trials in the areas of ischemic stroke, renal failure and diabetes. Irina holds a medical degree from Russia, and MSc in Pharmaceutical medicine from University of Surrey, UK.

PARTNER

Machaon Diagnostics
Coagulation, platelets, rare disease and genetics

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

WHY PARTNER

Are you front of mind when pharma and biotech look to develop commercially viable next generation hemophilia therapeutics?

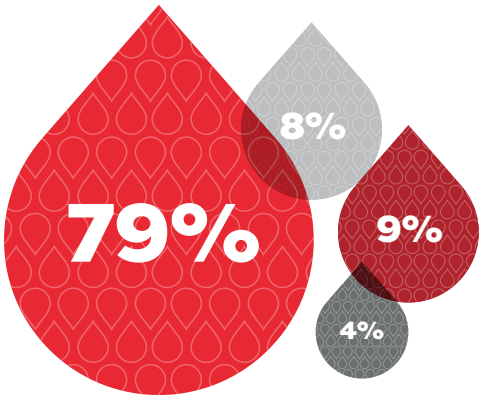
Key hemophilia drug developers have informed us that one of the most pressing problems in this rapidly growing field is that service providers lack knowledge and experience of the industry, forcing developers to use time and resources on work that they would rather outsource.

This is the perfect opportunity to demonstrate that you have the desire and capability to meet the increasing demands of this emerging field, and cement your position as the industry leader.

ATTENDING COMPANIES INCLUDE:

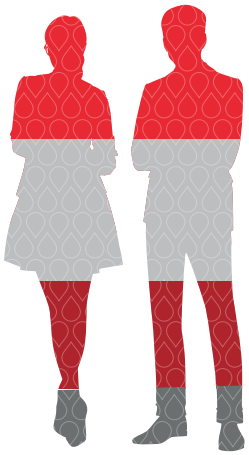
Bioverativ
CATALYST BIOSCIENCES
CSL Behring
novo nordisk
SANOFI GENZYME
uniQure
Spark THERAPEUTICS
BAYER

TYPICAL ATTENDANCE BY INDUSTRY TYPE:



- **Drug Developers**
- **Academics**
- **Solution and Service Providers**
- **Investors and Analysts**

OUR TYPICAL ATTENDANCE SENIORITY IN CVI:



- **C-level Executives: 12%**
- **Director Level and Above: 50%**
- **Head of: 20%**
- **Scientist: 13%**

*Based on market research and previous events' statistics

BECOME A PARTNER

Jennifer MacKay
Partnerships Director
T: +1 617 455 4188
E: sponsor@hansonwade.com

READY TO REGISTER?

3 EASY WAYS TO BOOK

www.hemophilia-drug-development.com

Tel: +1 617 455 4188

Email: register@hansonwade.com

Top 3 Benefits of Attending:

1. Engage with the leading experts in pharma and biotech in an intimate environment
2. Learn about the approaches taken by pioneers in the field to address key industry challenges
3. Immerse yourself in in-depth discussions on focused and relevant topics

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.

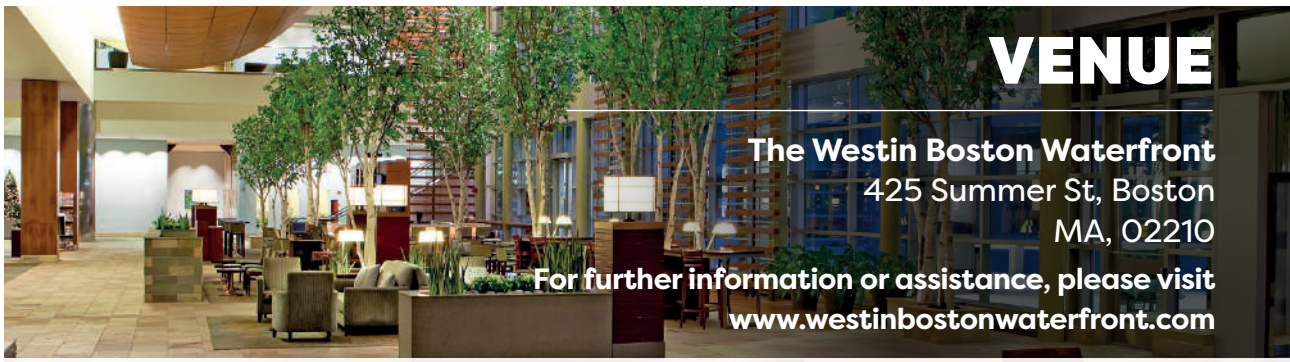
Contact: register@hansonwade.com

SECURE YOUR PLACE

Drug Developer (Pharma & Biotech)	Register & pay by Friday, May 11	Standard Prices
Conference + 2 Workshops	$2597 **(save $800)**	$3,197.00
Conference + 1 Workshop	$1998 **(save $700)**	$2,598.00
Conference Only	$1399 **(save $600)**	$1,999.00
Workshops (Each)	$699	

Academic Research Institute & Not-For-Profit	Register & pay by Friday, May 11	Standard Prices
Conference + 2 Workshops	$1948 **(save $670)**	$2,398
Conference + 1 Workshop	$1499 **(save $560)**	$1,949
Conference Only	$1049 **(save $450)**	$1,499
Workshops (Each)	$559	

Solution & Service Provider	Register & pay by Friday, May 11	Standard Prices
Conference + 2 Workshops	$3,097.00	$3,897.00
Conference + 1 Workshop	$2,598.00	$3,298.00
Conference Only	$2,099.00	$2,699.00
Workshops (Each)	$699	



VENUE

The Westin Boston Waterfront
425 Summer St, Boston
MA, 02210

For further information or assistance, please visit www.westinbostonwaterfront.com

Code: 8149