1ST WFH GENE THERAPY ROUND TABLE
Understanding the Landscape and Challenges of Gene Therapy for Hemophilia around the World

APRIL 5–6, 2018 · MONTREAL, CANADA
The 1st WFH Gene Therapy Round Table will bring together for the first time, WFH national member organizations, healthcare professionals (HCP), regulators, and industry for a global dialogue on the expected challenges and opportunities of gene therapy for hemophilia.

Objectives

• Understand the challenges associated with the development of gene therapy for the global hemophilia community
• Explore possible models of access to gene therapy that advance the WFH’s vision of Treatment for All
• Identify unmet educational opportunities and advocacy initiatives regarding gene therapy
• Develop strategies to address these opportunities for people with hemophilia, WFH national member organizations, and healthcare professionals

Program Committee

Chair
• Glenn Pierce, United States

Healthcare Professionals
• Marijke van den Berg, The Netherlands
• David Lillicrap, Canada
• John Pasi, United Kingdom
• Margareth Ozelo, Brazil
• Alok Srivastava, India

People with a bleeding disorder / caregiver
• Dawn Rotellini, United States
• Thomas Sannié, France
• Carlos Safadi Marquez, Argentina
April 5, 2018

OPENING: 8:00AM–9:00AM

Opening Remarks
Alain Weill, WFH President

Objectives of the Meeting
Moderator, Glenn Pierce

• Challenges & opportunities in gene therapy
  Glenn Pierce, USA

• The gene therapy pipeline
  John Pasi, UK

• Patient and HCP survey results
  Luisa Durante, WFH

Clinical Development and Regulatory Perspectives – Pre-authorization

SESSION 1: 9:00AM–10:15AM

Clinical Development of Gene Therapy
Moderator, John Pasi

• Clinical trial design
  • Clinical trial design aspects specific to gene therapy
    Lindsey George, USA
  • Challenges conducting gene therapy clinical trials in South Africa
    Johnny Mahlangu, South Africa
  • Challenges conducting gene therapy clinical trials in Brazil
    Margareth Ozelo, Brazil

• Patient perspective of participating in a gene therapy clinical trial
  Jack Grehan, UK

• Session 1 discussion

BREAK: 10:15AM–10:30AM · FOYER

SESSION 2: 10:30AM–12:00PM

Regulatory Perspectives of Gene Therapy
Moderator, Marijke van den Berg

• Measuring outcomes in gene therapy
  • coreHEM results
    Mark Skinner, USA

• Regulatory perspectives from FDA
  Jay Lozier, USA

• Regulatory perspectives from EMA
  Sol Ruiz, Spain

• Regulatory aspects unique to hemophilia
  Anneliese Hilger, Germany

• Regulatory perspectives from South Africa
  Paul Ruff, South Africa

• Session 2 discussion

LUNCH: 12:00PM–1:00PM · ROOM: ST-ANTOINE A
Making Informed Decisions and Accessibility, Affordability and Pricing – Post-authorization

SESSION 3: 1:00PM–2:40PM

Making Informed Decisions Moderator, Alok Srivastava

HCP Perspective
• Making informed decisions: gene therapy vs currently available therapies Maggie Ragni, USA
• The unknowns of gene therapy Glenn Pierce, USA
• HCP perspectives from around the world
  • China Renchi Yang, China
  • Senegal Saliou Diop, Senegal

Patient/Caregiver Perspective
• Patient perspective on making an informed decision Thomas Sannié, France
• Caregiver perspective on making an informed decision Dawn Rotellini, USA

• Session 3 discussion

BREAK: 2:40PM–3:00PM · FOYER

SESSION 4: 3:00PM–4:30PM

Accessibility, Affordability and Pricing Moderator, Jamie O’Hara

• Specific issues related to assessing the value of gene therapy Bengt Jönsson, Sweden
• Possible gene therapy payment models and reimbursement scenarios Mathias Flume, Germany
• Affordability of gene therapy in India Alok Srivastava, India
• The Gilead & HCV experience in Egypt: parallels to gene therapy and hemophilia Magdy El Ekiaby, Egypt

• Session 4 discussion

GROUP DISCUSSION: 4:30PM–5:30PM

Consolidating Discussion Moderator, David Lillicrap

• Identifying priority barriers and opportunities for gene therapy in hemophilia in various contexts

DINNER: 6:30PM · ROOM: VICTORIA, 3RD FLOOR
April 6, 2018

Advocacy and Moving Forward
– Enhancing the Understanding of Gene Therapy

**SESSION 5: 8:30AM–9:00AM**

**Ethics of Gene Therapy**
- Unique ethical concerns associated with gene therapy

**Moderator, Glenn Pierce**
Jonathan Kimmelman, Canada

- Session 5 discussion

**SUMMARY OF DAY 1: 9:00AM–9:30AM**

**Summary of Barriers and Opportunities Identified**

**Moderator, David Lillicrap**

**SMALL GROUP BREAK-OUT: 9:30AM–10:45AM**

**Addressing Barriers and Opportunities**
- Advocacy point of view
- Educational point of view
- Communicating barriers and opportunities to the community

**Moderator, Donna Coffin**
Group facilitators

**BREAK: 10:45AM–11:15AM · FOYER**

**BREAK-OUT SESSION REPORT: 11:15AM–12:30PM**

**Approaching Barriers and Opportunities**
- Advocacy point of view
- Educational point of view
- Advocacy and education tools

**Moderator, John Pasi**
Spokespeople from each group

**CLOSING: 12:30PM–1:00PM**

**Conclusions and Next Steps**
Glenn Pierce

**LUNCH: 1:00PM · GRAB N’ GO**
The 1st WFH Gene Therapy Round Table is supported by funding from:

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