



# 1<sup>ST</sup> WFH GENE THERAPY ROUND TABLE

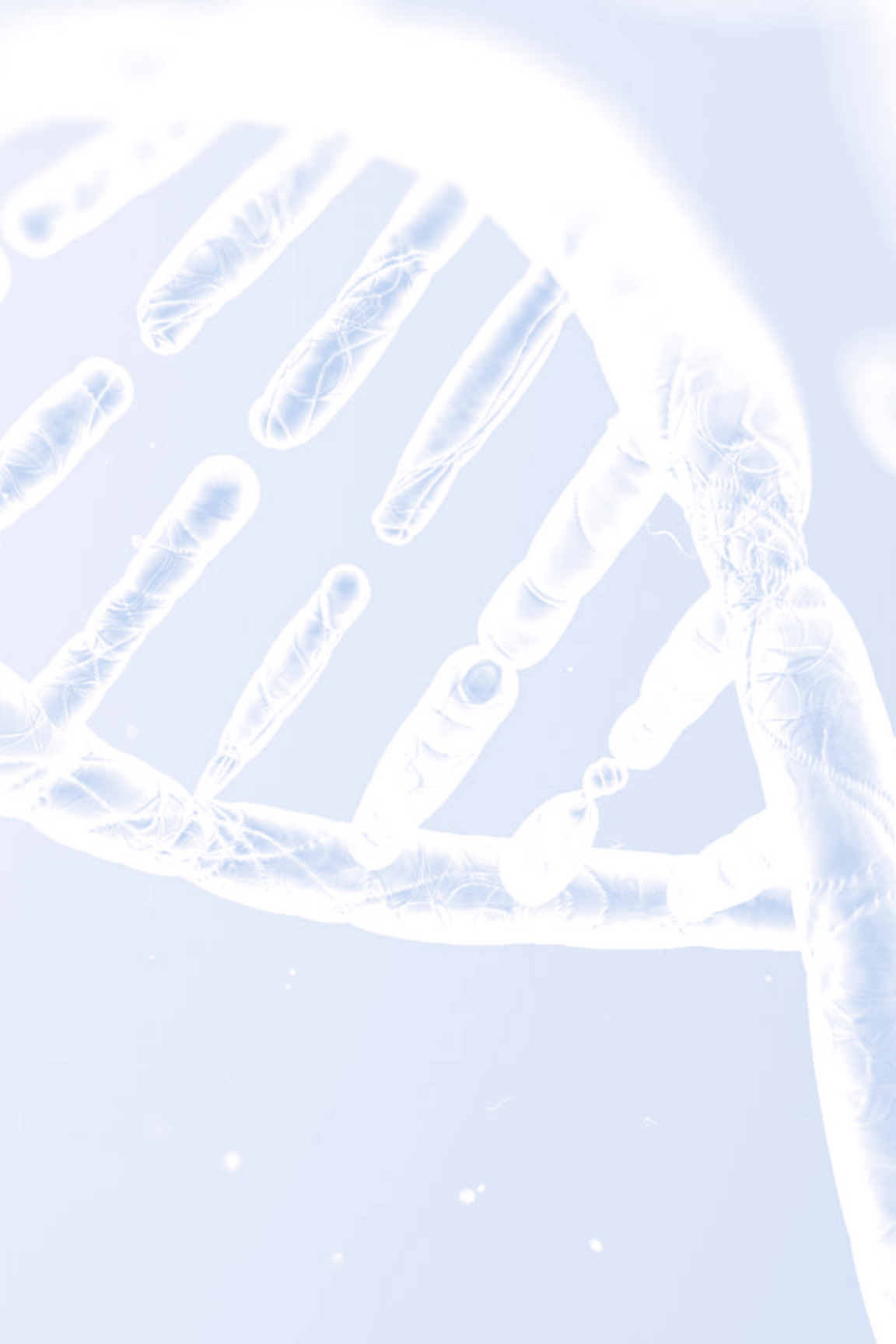
Understanding the Landscape and  
Challenges of Gene Therapy for  
Hemophilia around the World

**APRIL 5–6, 2018 · MONTREAL, CANADA**



**WFH**

WORLD FEDERATION OF HEMOPHILIA  
FÉDÉRATION MONDIALE DE L'HÉMOFILIE  
FEDERACIÓN MUNDIAL DE HEMOFILIA



The **1<sup>st</sup> 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

ROOM: VILLE-MARIE, 9TH FLOOR

## 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
- 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

ROOM: VILLE-MARIE, 9TH FLOOR

## 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

Moderator, Donna Coffin

- Advocacy point of view
- Educational point of view
- Communicating barriers and opportunities to the community

Group facilitators

**BREAK: 10:45AM–11:15AM · FOYER**

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

#### Approaching Barriers and Opportunities

Moderator, John Pasi

- Advocacy point of view
- Educational point of view
- Advocacy and education tools

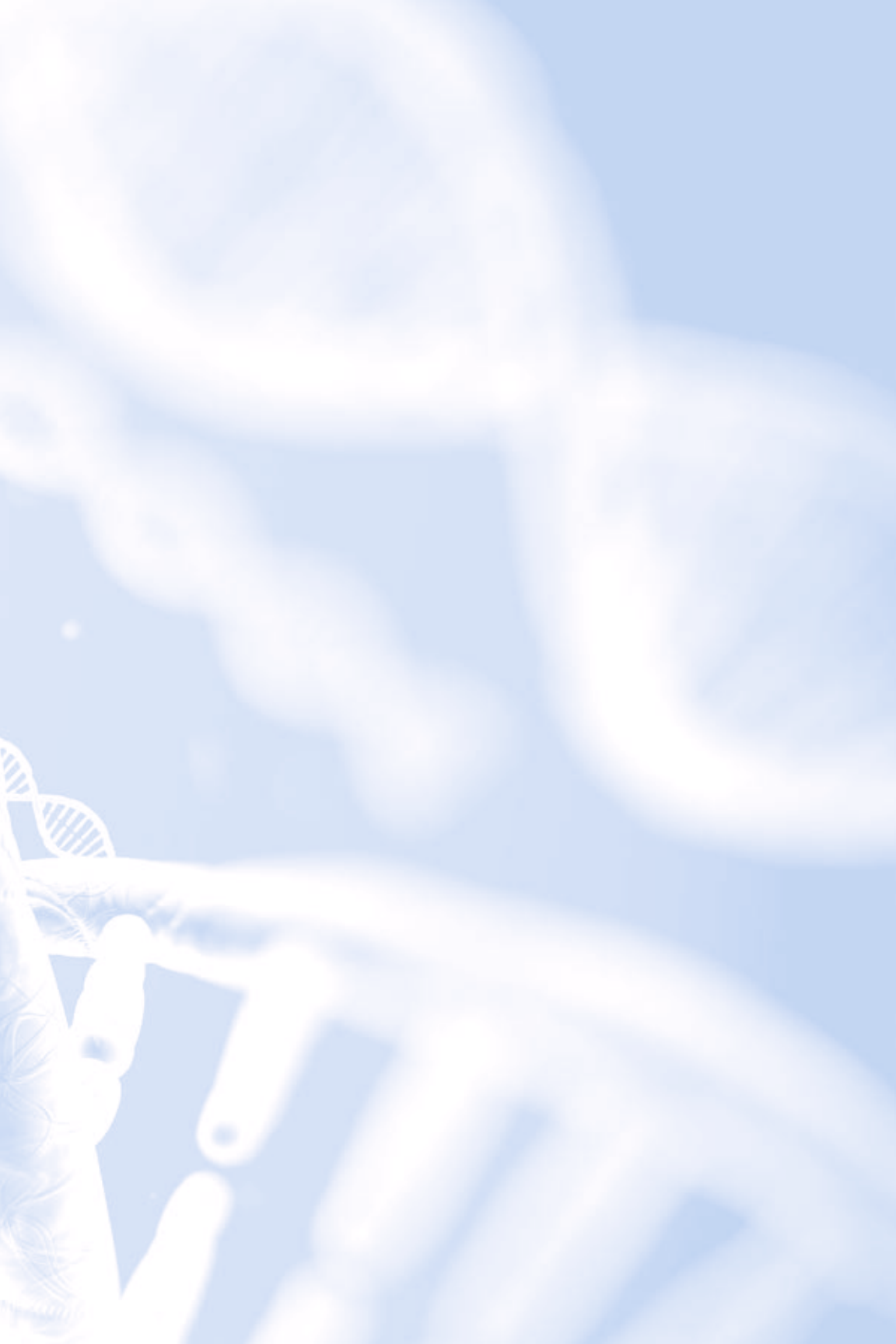
Spokespeople from each group

### CLOSING: 12:30PM–1:00PM

#### Conclusions and Next Steps

Glenn Pierce

**LUNCH: 1:00PM · GRAB N' GO**



The 1<sup>st</sup> WFH Gene Therapy Round Table  
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