# 5th WFH Gene Therapy Round Table
**November 17–18, 2022**  
**Montreal, Canada**

## NOVEMBER 17

### 8:30-8:40  
**Welcome**

### 8:40-9:00  
**Gene therapy update**  
- Gene therapy update - Radoslaw Kaczmarek
- How science is being communicated - Timothy Caulfield
- Science by press release for hemophilia - Mike Makris
- Editor perspective - importance of the peer review process, balanced data, and evidence - Cedric Hermans

### 9:00-10:30  
**Science by press release**  
- Factor 8 - Johnny Mahlangu
- Factor 9 - Wolfgang Miesbach
- Liver issues - Sylvia Fong

### 10:30-10:45  
**Break**

### 10:45-12:00  
**Safety**  
- Factor 8 - Margareth Castro Ozelo
- Factor 9 - Pratima Chowdary
- WFH Gene Therapy Registry - Barbara Konkle

### 12:00-13:00  
**Lunch**

### 13:00-14:15  
**Variability & durability**  
- REMS request in the USA - Len Valentino
- FDA perspective - Peter Marks
- EMA perspective - Andrea Laslop

### 14:15-14:30  
**Break**

### 14:30-16:00  
**Regulatory perspective**  
- Issues to consider for informed consent - HCP perspective - Lindsey George
- Issues to consider for informed consent - PWH perspective - Declan Noone
- WFH shared decision making tool - Courtney Thornburg
- How regulators look at decisions making for gene therapy - Martina Schuessler-Lenz

### 16:00-17:15  
**Informed consent & shared decision making**  
- Eligibility for gene therapy - current limitations - Alok Srivastava
- Gene therapy in China: efficacy, safety data, and future plans - Lei Zhang
- Approaches to overcoming immune challenges - Gallia Levy
- Clinical trials of inhibitors (of history of) - Tara Robinson

### 19:00-21:30  
**Dinner**

## NOVEMBER 18

### 8:00-9:30  
**Expanding eligibility**  
- Lentivirus and ET3 - Chris Doering
- Gene editing - Leah Sabin
- Non-viral gene therapy - Jack Rychak

### 9:30-9:45  
**Break**

### 9:45-11:00  
**Alternatives to AAV**  
- Dealing with uncertainty - Anja Schiel
- Impact of different payment methods on price/health care system - Lesley Tilson

### 11:00-12:00  
**Pricing gene therapy**  
- Outcomes and monitoring in the context of payment - Kollet Koulianos

### 12:00-12:45  
**Lunch**

### 12:45-13:50  
**Plans for commercialized gene therapy in HMIC**  
- Perspective from Argentina - Daniela Neme
- Perspective from Brazil - Margareth Castro Ozelo
- Perspective from South Africa - Johnny Mahlangu
- Perspective from Turkey - Kaan Kavakli
- Differences between LMIC & HMIC - Bishesh Poudyal

### 13:50-14:00  
**Closing remarks**
The 5th WFH Gene Therapy Round Table is supported with funding from:

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