



The 3rd WFH Gene Therapy Round Table

30 September – 1 October 2020

Day 1 – September 30th, 2020

8:45-8:55 **Welcome remarks**

8:55-9:15 **Title: Gene Therapy Update**

Chair: Glenn Pierce

Topics:

- The gene therapy pipeline – *John Pasi*
- The knowns and unknowns of gene therapy – *Glenn Pierce*

9:15-10:45 **Title: Unresolved issues in gene therapy for hemophilia**

Chair: Radoslaw Kaczmarek

Moderator: David Lillicrap

Topics:

- Liver toxicities - Questions that can be addressed by liver biopsies –*Graham Foster*
- Durability and variability of factor levels –*David Lillicrap*
- Pre-existing antibodies –*Edward Tuddenham*
- Coagulation assay discrepancy: the path towards resolution –*Steffen Rosén*

Question & Answer

10:45-10:55 Break

10:55-11:25 **Chair: John Pasi**

Presentation of results – Elicitation: Factor Level & Durability – *Jamie O’Hara*

Q&A and Discussion

11:25-11:55 **Title: Approving gene therapy products**

Chair: Glenn Pierce

Moderator: Lindsey George

Topics:

- FDA – *Peter Marks*
- PEI – *Anneliese Hilger & Mirco Müller-Olling*

11:55-12:15 **Chair: Glenn Pierce**

Q&A and Discussion

12:15-12:30 Break

12:30-13:30 **Title: Collecting long-term follow-up data: WFH Gene Therapy Registry**

Chair: Johnny Mahlangu

Moderator: Declan Noone

Topics:

- WFH GTR – *Glenn Pierce*
- EMA perspectives – *Caroline Voltz-Girolt*
- ATHN-14 – *Mike Recht*

Question & Answer



Day 2 – October 1st, 2020

9:00-9:05 **Title: Welcome back – Dr. Glenn Pierce & Dr. John Pasi**

9:05-10:05 **Title: Buying into the promise of gene therapy - patient perspectives**

Chair: John Pasi

Moderator: Dawn Rotellini

Topics:

- Managing expectations - A hematologist perspective – *John Pasi*
- Yes! But I was not eligible – *David Page*
- I'm waiting for the next generation of gene therapy – *Garret Hayes*
- Yes! I'm happy to have received gene therapy – *Rob Schroeder*
- I'm interested to have gene therapy, but it is not available in my country – *Enrique Preza Hernandez*

10:05-11:05 **Title: Unravelling the ethics of gene therapy**

Chair: Lindsey George

Moderator: Johnny Mahlangu

Topics:

- Ethics of gene therapy –*Erika Kleiderman*
- The informing in 'Informed Consent' – *Lindsey George*

Question & Answer

11:05-11:20 Break

11:20-12:35 **Title: Access to gene therapy globally: thoughts from the field**

Chair: Thomas Sannié

Moderator: Alok Srivastava

Topics:

- Possible funding models of gene therapy in LMIC – *Johnny Mahlangu*
- Access through clinical trials in LMIC – *Andrew Davidoff*
- Developing gene therapy products in LMIC – *Alok Srivastava*
- Role of NMOs in accessing gene therapy – *Bradley Rayner*

Question & Answer

12:35-13:05 **Chair: Declan Noone**

Presentation of results & discussion – Elicitation: Willingness to pay – *Jamie O'Hara*

Q&A and Discussion

13:05-13:15 **Title: GTRT 2020 wrap-up – Glenn Pierce & John Pasi**

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