



The 4th WFH Gene Therapy Round Table – Webinar Series

18 March, 10 June, 16 September 2021

Thursday March 18, 2021

OPENING 9:00 AM – 9:15 AM

WELCOME AND OBJECTIVES

Dr. Glenn Pierce, *USA*, Dr. John Pasi, *UK*

9:15-9:45 AM

GENE THERAPY UPDATE

Safety, durability, variability, efficacy

Dr. Glenn Pierce, *USA*, Dr. John Pasi, *UK*

SESSION 1: 9:45 AM – 11:25 AM

GENE THERAPY AS A CHOICE: MAKING AN INFORMED DECISION

Moderator: Dawn Rotellini, *USA*, Johnny Mahlangu, *South Africa*

9:45-10:00 **Nurse perspective on patient decision making**

Sara Garcia Barcenilla, *Spain*

10:00-10:15 **Attributes to consider: The PAVING Study**

Eline van Overbeeke, *Belgium*

10:15-10:30 **Exigency: why some people with haemophilia have no interest in gene therapy**

Simon Fletcher, *UK*

10:30-10:45 **Gene therapy considerations in LIC**

Bishesh Poudyal, *Nepal*

10:45-10:55 **Q&A and DISCUSSION**

10:55-11:25 **Panel Discussion on decision making**

Brian O'Mahony, *Ireland*, Luke Pembroke, *UK*, Steven Scott, *South Africa*

BREAK 11:25 – 11:35 AM

SESSION 2: 11:35 AM – 12:45 PM

SMALL GROUP BREAK OUTS

Moderators: John Pasi, *UK*

11:35-11:40 **Introduction to small group break-out**

Donna Coffin, *Canada*

11:40-12:10 **Small group break out**

1. Delivering balanced information
2. What does the evidence say?
3. Treatment related clinical considerations
4. Patient clinical profile
5. Patient preferences
6. Weighing the factors
7. Low resource setting

12:10-12:45 **Reporting back**

CLOSING 12:45 AM – 13:00 PM

MARCH WEBINAR WRAP-UP

Dr. Glenn Pierce, *USA*, Dr. John Pasi, *UK*



Thursday June 10, 2021

OPENING 9:00 AM – 9:10 AM

WELCOME AND OBJECTIVES

Dr. Glenn Pierce, *USA*

9:10-9:25 AM

GENE THERAPY UPDATE

Status update on gene therapy clinical trials

Dr. John Pasi, *UK*

SESSION 1: 9:25 AM – 10:55 AM

How should I interpret emerging data?

Moderator: Glenn Pierce

9:25-9:40 **Assay description**

Dr. Radoslaw Kaczmarek, *USA*

9:40-9:55 **What do the assays mean?**

Dr. David Lillicrap, *Canada*

9:55-10:05 **Discussion**

10:05-11:00 **What do the numbers mean? Panel & discussion**
Does the average patient exist? What numbers does each patient need to make an informed decision? How will I define whether gene therapy is working for me?

Dr. John Pasi, *UK*, Dr. Courtney Thornburg, *USA*, Dr. Shannon Jackson, *Canada*, Thomas Sannié, *France*, Declan Noone, *Ireland*, Mark Skinner, *USA*

BREAK 11:00 - 11:10 AM

SESSION 2: 11:10 AM – 12:40 PM

Looking into the crystal ball

Moderator: Johnny Mahlangu

11:10-11:25 **Organization & delivery of care – USA**

Dr. Steven Pipe, *USA*

11:25-11:40 **Organization & delivery of care – Europe**

Dr. Wolfgang Miesbach, *Germany*

11:40-11:55 **Organization & delivery of care – Brazil**

Dr. Margareth Ozelo, *Brazil*

11:55-12:10 **Discussion**

12:10-12:25 **Gene editing & hemophilia: possible or not?**

Dr. Robert Peters, *USA*

12:25-12:40 **Discussion**

CLOSING 12:40 PM – 12:55 PM

WEBINAR WRAP-UP

Dr. Glenn Pierce, *USA*, Dr. John Pasi, *UK*

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