



AGENDA

Wednesday, November 13, 2019

SAFETY, SUPPLY AND ACCESS TO TREATMENT PRODUCTS

8:30 – 8:40

WELCOMING REMARKS

Alain Weill

8:40 – 9:40

SESSION TBD

9:40 – 10:40

PLASMA-DERIVED PRODUCT RISKS

- Blood and plasma product safety. Mike Busch
- Safety and supply of plasma derived and recombinant products (not inhibitors)
- Update on products for the other rare bleeding disorders (FV concentrate and others)

EXTENDED HALF-LIFE PRODUCTS

- Inhibitors (lower immunogenicity of EHLs; new methods of tolerance induction)
- Per capita FVIII usage when more countries are buying EHL products and emicizumab

BREAK 10:40 – 11:00

11:00 – 12:00

NOVEL PRODUCTS

- Hemilibra and Fitusiran (and any other pipeline products). Measurement and assessment of these novel products
- Cell delivery of FVIII or FIX
- Vioxx for hemophilia patients (under product development)
- Subcutaneous FIX recombinant

LUNCH 12:00 – 13:30

13:30 – 14:30

ENSURING ACCESS TO AFFORDABLE TREATMENT PRODUCTS AND HUMANITARIAN AID

- Strategies to Increase Access to Care Session: HA and strategies for low cost factor
- WFH Humanitarian Aid
- GAP update
- PARTNERS update

14:30 – 15:10

RISKS TO TRIAL PARTICIPANTS

- Subcutaneous administration of CFC and immunogenicity
- Routes of immunization

BREAK 15:10 – 15:30

15:30 – 16:30

PANEL DISCUSSION TBD

16:30 – 17:30

GENE THERAPY

- Short update on gene therapy.
- Target levels post-therapy in the context of joint health.
- Report from 2nd WFH Gene Therapy Roundtable including topics that deserve broader dissemination.)
- Gene editing. Sync finger nuclease.
- Different assays and measurements.
- Gene therapy for non-hemophilia A or B. FV or FX def. What is the regulatory pathway?

DINNER 18:30 – 20:30

Thursday, November 14, 2019

RESEARCH AND HEMOPHILIA TREATMENT

8:30 – 10:30

NEW AND NOVEL HEMOPHILIA THERAPIES: LATEST UPDATES FROM CLINICAL TRIALS

BREAK 10:30 – 11:00

11:00 – 12:00

CONTRADICTORY INDICATIONS REGARDING PEG PRODUCTS

LUNCH 12:00 – 13:30

13:30 – 14:10

DATA COLLECTION

- EMA move from PMS to registries. Long term follow-up.
- WFH World Bleeding Disorders Registry

14:10 – 14:50

WFH RESEARCH PROGRAM

- AGS 20th anniversary.

BREAK 14:50 – 15:20

15:20 – 16:15

CLOSING SESSION TBD

CLOSE OF MEETING 16:15