

SELECTED TOPICS FOR THE PROGRAMME

GENE THERAPY WITH ADENO-ASSOCIATED VIRAL VECTORS

Chairs: Wolfgang Miesbach (Germany), Flora Peyvandi (Italy)

- State of the art in haemophilia and other rare diseases – Wolfgang Miesbach (Germany)
- Adeno-Associated Virus (AAV)-host interactions and AAV vector development – Hildegard Büning (Germany)
- AAV integration into genome: evolution of liver toxicity and cancerogenesis – tbd

OTHER STRATEGIES FOR GENE THERAPY

Chairs: Peter Lenting (France), David Lillicrap (Canada)

- Lentiviral vector transduced autologous hematopoietic stem cell-based gene therapy for haemophilia A – Alok Srivastava (India)
- Lentivirus in haemophilia B – Luigi Naldini (Italy)
- Non-viral strategies for gene therapy – David Lillicrap (Canada)

NEW CLINICAL ASPECTS

Chairs: Pier Mannuccio Mannucci (Italy), Mike Makris (UK)

- The use of antithrombotic drugs in haemophilia - Roger Schutgens (The Netherlands)
- Prophylaxis in infants with haemophilia A/B using bispecific antibodies - Karin Fijviandraat (The Netherlands)
- The clinician's choice: which patient is suitable for which therapy? Steven Pipe (US)

JOINT AND BONE HEALTH IN HEMOPHILIAS

Chairs: Roberta Gualtierotti (Italy), Raimondo De Cristofaro (Italy)

Open questions for joint/bone health

Telemedicine for joint health – Sergio Mascetti (Italy)

The role of factor VIII in bone physiology – Raimondo De Cristofaro (Italy)

NEW INDICATIONS FOR BISPECIFIC ANTIBODIES

Chairs: Caterina Casari (France), Cecile Denis (France)

- Bispecific antibodies in acquired haemophilia A – Andreas Tiede (Germany)
- Experimental and clinical rationale for the use of bispecific antibodies in von Willebrand disease – Caterina Casari (France)

LECTURES

- The use of antiangiogenic factors in Hereditary Hemorrhagic Telangiectasia HHT and their potential for use in VWD related angiodysplasia – Hanne Al Samkari (US)
- Von Willebrand factor functions outside haemostasis – James O'Donnell (Ireland)
- The loss of tolerance in patients who previously underwent ITI – Sébastien Lacroix-Desmazes (France)

ADVANCES IN THROMBOTIC MICROANGIOPATHIES (TMAs)

Chairs: Paul Coppo (France), Nicola Ciavarella (Italy)

- Novel mechanism underlying pathogenesis of immune thrombotic thrombocytopenic purpura (TTP) – Long Zheng (US)
- Anti-vWF therapies in iTTP: now and then – Paul Coppo (France)
- Challenges in the classification and nomenclature of different forms of hemolytic uremic syndrome – Marina Vivarelli (Italy)