

The future of Haemophilia: From Novel Factor Replacement to Beyond-Replacement Strategies

FAD SINCRONA, 13 novembre 2026 ore 14.00-18.10

Nome ed id. del provider: BIBA TOUR

ID PROVIDER: 1420

ID ECM:

Ore formative: 4

Destinatari dell'attività formativa: 200 PARTECIPANTI Biologi, Chimico, Farmacista Infermiere, Psicologo, Tecnico sanitario di Laboratorio Biomedico Medico Chirurgo: Ematologia, Farmacologia e tossicologia clinica, Genetica medica, Medicina Interna, Medicina Trasfusionale, Patologia clinica (lab. di analisi chimico-cliniche e microbiologia) Pediatria, Psicoterapia, Laboratorio di genetica medica

Obiettivo formativo: Obiettivo formativo tecnico-professionale n°2 – Linee guida protocolli e procedure

Responsabile Scientifico *C. Hermans, M. Napolitano, A. Maggio*

LINK FAD <http://www.bibagroup.it/eventi-biba/>

SEDE DELLA PIATTAFORMA Via Emilia, 38 – 90144 Palermo

Razionale

The therapeutic landscape of hemophilia is undergoing a profound transformation. While the field is rapidly expanding beyond conventional replacement therapy toward rebalancing strategies, gene therapy, and gene editing, replacement treatment itself is also entering a new phase of innovation.

In particular, the availability of novel factor replacement approaches, including high-sustained factor VIII therapies such as efanesoctocog alfa, is reshaping expectations in prophylaxis, bleed protection, treatment personalization, and quality of life. These advances are broadening the clinical possibilities within replacement therapy and are redefining how clinicians position factor-based strategies in relation to non-replacement options.

At the same time, the emergence of rebalancing therapies, RNA interference approaches, AAV-based gene therapy, and gene editing is creating an increasingly complex decision-making environment for hemophilia treatment centers. Key issues now include treatment selection, patient profiling, monitoring, long-term safety, durability of response, access, sustainability, and future care pathways.

Webinar Garden 2026 aims to provide a multidisciplinary update on the most recent advances, integrating clinical evidence, real-world data, international consensus recommendations, and the patient perspective, with the goal of outlining the future management of coagulation disorders in the era of advanced therapies.

Programma

2:00-2:10 pm – Opening & Welcome

M. Napolitano (Italy), A. Maggio (Italy)

Presentation of objectives

Overview of the 2025–2026 paradigm shift

2:10-3:00 pm – SESSION I

The new therapeutic Era

Chair: C. Hermans (Netherlands), M. Napolitano (Italy)

The Real-World Evolution of Hemophilia Care

M. Napolitano (Italy)

Beyond Factor Replacement: Clinical Trial Evidence and Real-World Challenges of Rebalancing Strategies.

Guy Young (USA)

Substitute: Maria Elisa Mancuso / Pratima Chowdary

Discussion

3:00-4:10 pm – SESSION II

Gene Therapy: From Trials to Clinical Practice

Chair: M. Napolitano

Valoctocogene and Etranacogene in 2026: Durability, Immunogenicity and Patient Selection
Sarina Levy (Israel)



Gene Therapy in the Real World: Building Sustainable Long-Term Follow-Up Models Wolfgang Miesbach / Glenn Pierce

Discussion

4:10-4:25 pm – VIRTUAL BREAK

4:25-5:10 pm – SESSION III

Gene Editing & Lentiviral Approaches as Curative Strategies

Chair: S. Levy (Israel)

Gene Editing for Hemophilia A: From Intron 22 Inversion to Base Editing
M. Pinotti (Italy)

Lentiviral Gene Therapy with CD34+ Hematopoietic Cells for Hemophilia A
A. Srivastava (India)

Discussion

5:10-5:30 pm – SESSION IV

Consensus, Complexity and New Clinical Scenarios

Chair: A. Srivastava (India)

2026 International Consensus: Managing Hemophilia in the Era of Advanced Therapies C.
Hermans (Netherlands)

5:30-6:10 pm – FINAL ROUND TABLE SESSION Is Hemophilia Still a Rare Disease in 2030? Participants:

Chairs

Gene Therapy Expert

Rebalancing Therapy Clinician

Patient Association Representative Health Policy Expert

6.10 pm CLOSING REMARKS

NOME	COGNOME	PROFESSIONE	DISCIPLINA	ENTE DI APPARTENENZA/LIBERA PROFESSIONE	DESCRIZIONE ATTIVITA' PROFESSIONALE
Aurelio	Maggio	Medico Chirurgo	Ematologia	Presidente Fondazione Franco e Piera Cutino ETS, Palermo, IT	Retired
Mariasanta	Napolitano	Medico Chirurgo	Ematologia	Haematology and Rare Disease Univeristy Unit, Hospital "V.Cervello", Palermo, IT	Head of the Haematology and Professor of Hematology, Director of hematology, University of Palermo
Cedric	Hermans	Medico Chirurgo	Coagulation Disorders	Division of Haematology Cliniques universitaires Saint-Luc Catholic University of Louvain, Brussels, BELGIUM	Full Professor, Head Haematology

Maria Elisa	Mancuso	Medico Chirurgo	Ematologia	Center for Thrombosis and Hemorrhagic Diseases, IRCCS Humanitas Research Hospital/Fondazione IRCCS Ca' Granda – Ospedale Maggiore Policlinico, Rozzano, Milan, IT	Senior Hematology Consultant, Bonomi Hemophilia and Thrombosis Center
Pratima	Chowdary	Medico Chirurgo	Ematologia	Department of Haematology, Cancer Institute, University College London, London, UK.	Professor of Haemostasis and Thrombosis
Guy	Young	Medico Chirurgo	Pediatric hematology/oncology	Children's Hospital Los Angeles, US	Pediatric hematologist at Children's Hospital, professor of pediatrics at the School of Medicine
Kathelijin	Fischer	Medico Chirurgo	Paediatric haematology/oncology	Center for Benign Haematology, Thrombosis and Haemostasis, Van Creveldkliniek, University Medical Center Utrecht, University Utrecht, Utrecht, the Netherlands	Paediatric Haematologist, Clinical Principal Investigator
Glenn	Pierce	Medico Chirurgo	Pathology and hematology	World Federation of Hemophilia, Montreal, QC, Canada	Physician-researcher, independent consultant
Wolfgang	Miesbach	Medico Chirurgo	Ematologia	University Hospital Frankfurt, Frankfurt am Main, DE	Professor of Medicine and Hematology, Head of Hemophilia and Haemophilia Center of the Hospital
Sarina	Levy-Mendelovich	Medico Chirurgo	Paediatric haematology/oncology	The National Hemophilia Center, and Amalia Biron Research Institute of Thrombosis and Hemostasis, Sheba Medical Center, Ramat Gan, Israel Tel Aviv, HaMerkaz, ISR	Pediatric hemato-oncologist, She works as a senior physician in a hemophilia thrombosis center, Israel and faculty of medicine
Flora	Peyvandi	Medico Chirurgo	Fisiopatologia	Università degli studi di Milano	DIRETTORE DI DIPARTIMENTO Medico-Chirurgica e dei Trapianti
Catherine	Lambert	Medico Chirurgo	Hematology, bleeding and thrombotic disorders	Cliniques Universitaires Saint-Luc, Bruxelles, Belgium	Head of Adult Hematology Department, Thrombose- Thrombopénie e
Mirko	Pinotti	Biologo	Biologia, Dottorato di Ricerca Biochimica	Università di Ferrara, IT	Direttore Dipartimento – Member of the European Society of Thrombosis and Haemostasis, Coordinatore del Corso di Laurea in Scienze della Medicina, coordinatore di Dottorati di Ricerca

Il provider, ai sensi dall' art. 47 del DPR n.445/2000, consapevole delle conseguenze previste dall'art. 76, dichiara:

- di aver fornito all'interessato l'informativa sul trattamento dei dati personali (art. 13 del Regolamento europeo 2016/679; artt. 68, 70, 76, 96 Accordo Stato-Regioni 2017 "La formazione continua nel settore salute"- Rep. Atti 14/CSR del 2.2.2017 - Par. 4.6, lett. j) Manuale Nazionale di Accreditamento per l'Erogazione di Eventi ECM);
- di aver informato l'interessato che il programma dell'evento ECM, di cui le suddette informazioni contribuiscono a formarne il contenuto minimo, verrà inserito nel catalogo degli eventi E.C.M. tenuto dall'ente accreditante;