GARDEN WEBINARS: A COMPRENSIVE APPROACH FOR BREAKING-UP THE COMMON BARRIERS OF HEMATOLOGICAL RARE DISEASES

ADVANCED TREATMENTS IN THE REAL-LIFE OF COAGULATION AND RED CELLS RARE DISORDERS

FAD SINCRONA ECM

06 SETTEMBRE 2025

DATE DI SVOLGIMENTO: 06 SETTEMBRE 2025

ORE FORMATIVE: 6 ore

N. PARTECIPANTI: 500

ID ECM: 275 - 455493

CREDITI ECM PREVISTI: 9

URL PIATTAFORMA FAD: https://infomed-ecm.it/

SEDE PIATTAFORMA FAD: Via San Gregorio 12 – 20124 Milano

RESPONSABILE SCIENTIFICO
Prof. A. Maggio

COMITATO SCIENTIFICO:
A. Maggio, Cedric Hermans, Ali Taher

OBIETTIVO FORMATIVO: 3 - Documentazione clinica. Percorsi clinico-assistenziali diagnostici e riabilitativi, profili di assistenza - profili di cura

RAZIONALE

Rare hematological diseases, while individually uncommon, collectively impact millions of lives worldwide. These conditions present significant challenges in terms of early diagnosis, treatment accessibility, and appropriate and effective patient communication. Overcoming these barriers requires a multidisciplinary international network aimed at establishing a common framework for corrective actions.
The rapid evolution of treatment options for rare hematological diseases necessitates ongoing education for healthcare professionals. This event focuses on several key areas where recent progress has been made, including:

- Hemophilia: The program will explore real-world data on current hemophilia treatments and prophylaxis, including the latest international consensus recommendations. This is crucial for optimizing patient outcomes and ensuring consistent standards of care.

- Thalassemia and MDS: The event will address the real-life experience with luspatercept, a novel therapeutic agent, in the management of these challenging conditions. Sharing real-world data is essential for understanding the drug's effectiveness and safety profile in diverse patient populations;

- PK Deficiency, Thalassemia, and SCD: The program will highlight the role of mitapivat and etavopivat in these distinct diseases, providing a unique opportunity to compare and contrast its application across different rare hematological conditions. This comparative approach will enhance participants' understanding of targeted therapies.
- PTT: this is a real hematological emergency and difficulty in diagnosis and appropriate treatment recommendations in the "real-life" are so far to be empowered.
- PNH: the patients with this very rare hematological disease (1/1.000.000 inhabitants) now are living with the possibility of having different treatments. However, appropriate information and costs of these innovative treatments may become barriers for their use.
- GENE THERAPY: is now at the "the patient's bedside" in some European countries. The meeting will address data from real-life in UK.
- AI: Artificial Intelligence may change our approach in collecting data of patients with rare hematological diseases. This educational will address the possible use of a new AI tool to collect in more appropriate and faster way clinical data for randomized clinical trials.

TARGET DEL CORSO:

Medico Chirurgo
- Ematologia
- Oncologia

Biologo
- Biologo

Tecnico sanitario laboratorio biomedico
- Tecnico sanitario laboratorio biomedico

Infermiere
- Infermiere

OBIETTIVO:

L'obiettivo di questo evento educativo è migliorare le conoscenze e le competenze dei partecipanti nella gestione delle malattie ematologiche rare. Diffondendo i più recenti progressi terapeutici e promuovendo la collaborazione tra i diversi attori coinvolti, si punta a elevare la qualità dell'assistenza e a ottimizzare gli esiti per i pazienti.

PROGRAMMA SCIENTIFICO

09:00 – **Introduction** – A. Maggio

09:10 - 10:40 - FIRST SESSION
Chairs: C. Hermans e M. Napolitano

09:10 – 09:30 Treatments for Haemophilia; data from the "real life"
M. Napolitano
09:30 – 09:50 Current prophylaxis treatment for severe haemophilia Haemophilia A
M. K. Economou
09:50 – 10:10 International consensus recommendations on the management of people with Haemophilia
C. Hermans
10:10 – 10:30 Highlights and concerns in TTP treatment
R. De Cristofaro
10:30 – 10:40 Q & A session:
ALL FACULTY

10:40 - 11:50 – SECOND SESSION
Chair: A. Taher

10:40 – 11:00 Real-life data on Luspatercept in Thalassemia
M.D. Cappellini
11:00 – 11:20 Real-life data on Luspatercept in MDS
M. Della Porta
11:20 – 11:50 Q & A session:
ALL FACULTY

11:50 - 13:15 – THIRD SESSION
Chair: K. Kuo

11:50 – 12:10 Mitapivat in Thalassemia
K. Musallam
12:10 – 12:30 Mitapivat in PK deficiency
K. Kuo
12:30 – 12:50 Mitapivat and Etavopivat in SCD
M. Abboud
12:50 – 13:15 Q & A session
ALL FACULTY

13:15 - 14:00
Break

14:00 - 14:40 – FOURTH SESSION
Chair: L. Luzzatto

14:00 – 14:15 Pathophysiology of PNH as target for innovative treatment
R. Notaro
14:15 – 14:30 Current and ongoing innovative treatments in PNH
E. Solomou
14:30 – 14:40 Q & A session
ALL FACULTY

14:40 - 14:50 – FIFTH SESSION
Chair: A. Maggio

14:40 - 14:50 Gene Therapy in Thalassemia and SCD: data from the real-life in UK
J. De La Fuente

14:50 - 15:00 – SIXTH SESSION
Chair: G. Valentini

14:50 – 15:00 Artificial Intelligence assisted prescreening for trial eligibility: possible application in rare hematological diseases
A.J. Blood

15:00 - 16:00 – SEVENTH SESSION
Chairs: M.K. Economou e D. Martino

15:00 – 16:00 ROUND TABLE: Exploring the barriers and promoting actions together
Associations of the patients, HTA experts, Psychologists, Institutional Representatives and the panel of experts
ALL FACULTY

16:00 - 16:15 Closing remarks
A. Maggio