



GARDEN
GLOBAL HEMATOLOGICAL
RARE DISEASES ALLIANCE



FAD SINCRONA ECM

6 | **Settembre**
2025

Ore 09.00-16.15

Advanced Treatments in the real-life of **Coagulation** and **Red Cells** Rare Disorders

Responsabile Scientifico: Aurelio Maggio

GARDEN
WEBINARS
SERIES

a comprehensive approach
for breaking-up
the common barriers of
hematological
rare
diseases



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GARDEN WEBINARS SERIES

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.

Responsabile Scientifico:
Aurelio Maggio

Comitato Scientifico:
Aurelio Maggio
Cedric Hermans
Ali Taher

a comprehensive approach for breaking-up the common barriers of hematological rare diseases

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

TTP: 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.

Advanced Treatments in the
real-life of **Coagulation** and
Red Cells Rare Disorders► 09.00 *Introduction - A. Maggio*

09.10-10.40

FIRST SESSION

Chairs: C. Hermans, 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: K. Musallam

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
A. Taher

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

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Advanced Treatments in the real-life of **Coagulation** and **Red Cells** Rare Disorders

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, 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

Faculty

Miguel Abboud, Lebanon

Alexander J. Blood, USA

Maria Domenica Cappellini, Italy

Raimondo De Cristofaro, Italy

Josu De La Fuente, UK

Matteo Della Porta, Italy

Marina Kelly Economou, Greece

Cedric Hermans, Belgium

Kevin Kuo, Canada

Lucio Luzzatto, Italy

Aurelio Maggio, Italy

Dario Martino, Italy

Khaled Musallam, UAE

Mariasanta Napolitano, Italy

Rosario Notaro, Italy

Elena Solomou, Greece

Ali Taher, Lebanon

Giorgio Valentini, Italy

ECM - EDUCAZIONE CONTINUA IN MEDICINA

Tipologia del Corso: **FAD Sincrona**

Durata formativa: **6 ore**

Crediti ECM: n. **9**

ID ECM n°: **275-455493**

Professioni:

- **Medico Chirurgo** (Ematologia, Oncologia)
- **Biologo**
- **Tecnico sanitario laboratorio biomedico**
- **Infermiere**

N. partecipanti previsti: **500**

Obiettivo Formativo: **3 - Documentazione clinica.**

Percorsi clinico-assistenziali diagnostici
e riabilitativi, profili di assistenza - profili di cura

Istruzioni per iscrizioni

- 1) Inquadrare il Qr Code o collegarsi al seguente link:
<https://infomed-ecm.it/event/1040/showCard>
- 2) Cliccare su **ISCRIVITI**
- 3) Cliccare su **LOGIN**
(se già registrato alla piattaforma Infomed)
ed effettuare il login con le proprie credenziali.
- 4) Cliccare su **REGISTRATI**
(se non iscritto alla piattaforma Infomed)
per compilare la scheda anagrafica
e procedere all'iscrizione all'evento.



Compilazione Questionario ECM

Al termine dell'incontro, i partecipanti dovranno sostenere un Test di Valutazione a risposta multipla e compilare il Modulo di Qualità Percepita tramite la piattaforma:

- numero massimo di tentativi a disposizione: 1
- soglia di superamento: 75% delle risposte corrette

Test di Valutazione e Modulo di Qualità Percepita dovranno essere compilati entro le ore 24.00 del 09/09/2025

PROVIDER ECM N. 275 e SEGRETERIA ORGANIZZATIVA



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