GARDEN
GLOBAL HEMATOLOGICAL RARE DISEASES ALLIANCE

Fondazione Franco e Piera Cutino
Guarire dalla TALASSEMIA

FAD SINCRONA ECM / SYNCHRONOUS CME WEBINAR

29 November 2025

Ore 09.00-13.45 (Italian Time)

The Role of Collaboration between Regulatory Agencies and Researchers in promoting innovation for Hematological Rare Diseases

Scientific Coordinator: Aurelio Maggio

GARDEN WEBINARS SERIES

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

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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 role of regulatory agencies in promoting innovation for hematological rare diseases is critical to improving patient outcomes through expedited development and access to novel therapies.

Scientific Coordinator:
Aurelio Maggio

Scientific Committee:
Aurelio Maggio
Stefano Rivella

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Their responsibilities include:

Facilitating Accelerated Approval Pathways: Agencies such as the FDA (U.S. Food and Drug Administration), EMA (European Medicines Agency), and others often implement programs like Breakthrough Therapy Designation, PRIME scheme, Priority Review, and Adaptive Pathways to fast-track promising treatments.

Providing Guidance and Support: They issue scientific advice, guidance documents, and frameworks to help researchers and pharmaceutical companies develop therapies tailored to the unique challenges of hematological rare diseases, such as limited patient populations and biomarker validation.

Encouraging Orphan Drug Designation: Regulatory agencies offer incentives like market exclusivity, tax credits, and grants for developing treatments targeted at rare diseases, thus fostering innovation by reducing financial risks.

Supporting Innovative Trial Designs: Promoting adaptive clinical trials, surrogate endpoints, and real-world evidence collection helps accelerate the evaluation process and adapt to the specific needs of rare hematological conditions

Promoting Global Collaboration: Agencies often engage in international cooperation to harmonize standards, share data, and streamline approval processes across borders, thereby increasing access to innovative therapies worldwide.

The Role of Collaboration between Regulatory Agencies and Researchers in promoting innovation for Hematological Rare Diseases

Particularly, EMA and National Regulatory Agencies across EU have established a robust ecosystem of support tools for developers, combining early engagement, regulatory guidance, collaborative networks, compliance resources, and training. These tools are designed to streamline the development and regulatory approval of innovative medicines and health technologies, with a strong emphasis on digital transformation and AI integration. Moreover, a positive benefit-risk balance is essential for marketing authorization. Therefore, Regulatory Agencies work to look at the process as dynamic, according to the evidence-driven, and ensuring that only medicines with a favorable balance of benefits over risks will be available to patients, with ongoing monitoring to safeguard public health. Finally, while drug development for rare diseases faces significant scientific, economic, and regulatory obstacles, Regulatory Agencies make opportunities possible through innovative use of data, technology, policy incentives, and collaborative models. Harnessing these strategies is essential to bring life-changing therapies to patients with rare conditions.

The rapid evolution of treatment options for rare hematological diseases necessitates ongoing education for healthcare professionals on the innovative treatments, on regulatory requirements and available support tools for developers, as well as about the approaches in determining cost vs benefit advantage and the regulation of new tool as AI. This event focuses on several key areas where recent progress has been made in term of academic approaches for the cure of thalassemia, hemophilia and disorders of iron pathways. A model for calculating the cost of gene therapies, according to the different epidemiology of the rare hematological disease in the single country will be presented. Finally, the EMA and EU Regulatory Network approaches to address the challenges posed by innovative technologies and methodologies applied to drug developments will be presented. These issues will be discussed with AIFA representatives who will share their perspective and available support tools for researchers to translate from academic research to the bed of the patient's product. At the end of the webinar, it will be held with the participation of the associations of the patients, the regulatory agencies, the participating KOL, psychologists and the HTA experts.

GARDEN D'AGOSTINO AWARD FOR INNOVATIVE RESEARCH IN HEMATOLOGICAL RARE DISEASES

The D'Agostino Prize for Innovative Research in Hematological Rare Diseases honors outstanding scientific contributions that advance the understanding, diagnosis, or treatment of rare blood disorders

This prestigious award recognizes groundbreaking research that demonstrates creativity, scientific rigor, and the potential to significantly impact patient outcomes in the field of hematology.

All details on the award are shown on **https://garden.fondazionecutino.it.**

The deadline for the presentation of the application is by October 6, 2025 at 2:00 pm.

29 November 2025

Ore 09.00-13.45 (Italian Time)

09.00-09.10
Introduction - A. Maggio

09.10-10.45

FIRST SESSION

Chair: S. Rivella

Challenges and opportunities of new treatments in Thalassemia, Hemophilia and Anemia: *setting the scene*

09.10-09.30
Reframing Thalassemia Syndrome as a benign haematopoietic stem-cell disorder
A. Maggio

09.30-09.50
Translating fundamental biology of erythropoiesis into the cure of Thalassemia
S. Rivella

09.50-10.10
Molecular approaches for controlling iron pathways to treat Anemia
O. Marques

10.10-10.30
Gene Editing approach for the cure of Hemophilia
M. Pinotti

10.30-10.45
Q&A session
All Faculty

The Role of Collaboration between Regulatory Agencies and Researchers in promoting innovation for Hematological Rare Diseases

10.45-12.45

SECOND SESSION

Chairs: P. Foggi (to be confirmed), **A. Maggio**

Challenges and opportunities of new treatments in Thalassemia, Hemophilia and Anemia: *understanding regulatory requirements and how to make the best use of new technologies*

10.45-11.10
Challenges and opportunities in drug development for rare diseases: regulators perspective
A. Isgrò *(to be confirmed)*

11.10-11.35
The role of regulators to support innovation and access to new treatments for rare diseases
P. Foggi *(to be confirmed)*

11.35-12.00
The regulatory approaches of EMA for implementing the appropriate use of AI in medicine
A. Magrelli *(to be confirmed)*

12.00-12.25
Opportunities and Challenges of Artificial Intelligence in Hematology
S. D'Amico

12.25-12.45
Q&A session
All Faculty

29 November 2025

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12.45-13.30

THIRD SESSION

Chair: A. Messori

How to translate these new approaches in possible drug for Thalassemia, Hemophilia, and Anemia

12.45-13.05
Inverse relation between the price of innovative treatments and disease incidence
A. Messori

13.05-13.25
Impact of joint scientific consultation and joint clinical evaluation on the future development and access to new drugs for rare diseases
P. Rivetti di Val Cervo (to be confirmed)

13.25-13.30
Q&A session
All Faculty

13.30-13.45

Garden D'Agostino Award Ceremony

13.45
Closing remarks - A. Maggio

The Role of Collaboration between Regulatory Agencies and Researchers in promoting innovation for Hematological Rare Diseases

Faculty

D'amico Saverio (Milano, ITA)
Foggi Paolo (Roma, ITA)
Isgrò Antonella (Roma, ITA)
Maggio Aurelio (Palermo, ITA)
Magrelli Armando (Roma, ITA)
Messori Andrea (Firenze,, ITA)
Paixao Marques Oriana (Heidelberg, Germany)
Pinotti Mirko (Ferrara, ITA)
Rivella Stefano (Philadelphia, USA)
Rivetti Di Val Cervo Pia (Roma, ITA)

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ECM - EDUCAZIONE CONTINUA IN MEDICINA

Tipologia del Corso: **FAD Sincrona**
Durata formativa: **5 ore**
Crediti ECM: n. **7,5**
ID ECM n°: **275-460423**
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

Please note that registration for the allocation of ECM credits is valid exclusively for italian residents.

1) Inquadrare il Qr Code o collegarsi al seguente link:
https://infomed-ecm.it/event/????/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 diposizione: 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 01/12/2025**

PROVIDER ECM N. 275 e SEGRETERIA ORGANIZZATIVA

Via San Gregorio, 12 - 20124 MILANO (Italy)
Tel. +39 02 49453331
giovanni.bondanini@infomed-online.it
www.infomed-online.it

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