

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

**THE ROLE OF ARTIFICIAL INTELLIGENCE IN THE DEVELOPMENT
OF DATA COLLECTION AND PROGNOSTIC FACTORS IN RARE
HEMATOLOGICAL DISEASES**

FAD SINCRONA ECM

24 APRIL 2026

DATE: April 24, 2026

TRAINING HOURS: 5 hours

NUMBER OF PARTICIPANTS: 500

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FAD PLATFORM URL: <https://infomed-eqm.it/>

FAD PLATFORM LOCATION: Via San Gregorio 12 – 20124 Milan, Italy

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TRAINING OBJECTIVE:

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

RAZIONALE

Rare hematological diseases, although individually uncommon, collectively affect millions of people worldwide. They share major challenges such as delayed diagnosis, limited access to treatments, and the need for effective patient communication.

Addressing these issues requires a multidisciplinary and international network committed to building a shared framework for action. The rapid evolution of therapies demands continuous education for healthcare professionals. This event is part of the GARDEN Network CME Webinar Series focusing on key areas of recent progress:

Hemophilia: Updates on real-world evidence and international consensus on prophylaxis and treatment to ensure consistent standards of care;

Thalassemia and MDS: Insights into the real-life use of luspatercept and its role in improving outcomes and safety across patient populations.;

- PK Deficiency, Thalassemia, and SCD: A comparative view on mitapivat and etavopivat as targeted therapies across different rare red cell disorders;
- TTP: Challenges in real-life diagnosis and management of this hematological emergency;
- PNH: Advances in available treatments and ongoing barriers linked to cost and access;
- GENE THERAPY: Real-world evidence from European experience, marking its arrival at the patient's bedside;
- AI: Exploring new AI tools to enhance clinical data collection and support future clinical trials in rare hematological diseases.

Artificial Intelligence (AI) is becoming an essential tool in medical research, offering unprecedented opportunities in data management, pattern recognition, and the development of prognostic models.

In the field of rare hematological diseases — where small patient populations and fragmented data pose significant challenges — AI can support clinicians and researchers in improving diagnosis, refining prognostic factors, and enhancing patient outcomes. This educational event will bring together international experts to present the current state of AI applications in hematology, share real-world experiences, and discuss the ethical and practical aspects of integrating AI into clinical research. A specific focus will be devoted to the role of AI in advancing a new vision of thalassemia as a

benign disorder of hematopoietic stem cells (HSCs). Through AI-assisted literature review and the creation of virtual patient cohorts within the GARDEN Network, it will be possible to strengthen the scientific evidence supporting this concept and to facilitate the design of more robust pharmacological trials. This methodology — already successfully applied to myelodysplastic syndromes — can be extended to other rare hematological diseases, promoting a unified data-driven research model. The ultimate goal of the webinar is to reach a consensus agreement among participants and to lay the foundation for an International GARDEN Consortium dedicated to conducting meta-analyses, building AI-supported virtual cohorts, and driving pharmacological innovation in rare hematological disorders.

COURSE TARGET AUDIENCE:

Medical Doctor

- Hematology
- Oncology

Biologist

- Biologist

Biomedical Laboratory Technician

- Biomedical Laboratory Technician

Nurse

- Nurse

Hematologists, Researchers, Associations of patients, Clinicians, Data Scientists, Nurses, Biologists, Healthcare Professionals involved in rare hematological diseases

OBJECTIVE:

The objective of this educational event is to enhance participants' knowledge and skills in the management of rare hematological diseases. By disseminating the latest therapeutic advances and promoting collaboration among the various stakeholders involved, the aim is to improve the quality of care and optimize patient outcomes.

SCIENTIFIC PROGRAM

14:00-14:10 Welcome and Introduction – Chair of the Scientific Committee – A. Maggio

14:10 - 15:10 - FIRST SESSION: Thalassemia Today from the Perspective of Conventional, Pharmacological, and Gene Therapy

Chair: Alok Srivastava

14:10 – 14:30 The approach to the current treatment in Thalassemia

M. D. Cappellini

14:30 – 14:50 The pharmacological approach for curing Thalassemia

A. Taher

14:50 – 15:10 Gene Addition and Gene Editing in Thalassemia: what we learnt by the real-life

J. De La Fuente

15:10 - 16:10 – SECOND SESSION: Artificial Intelligence Models for Rare Diseases: The Myelodysplasia and Myeloproliferative Model

Chair: L. De Angelis

15:10 – 15:30 The Synthema Model: Overcoming Data Fragmentation and Scarcity in Rare Hematological Diseases

F. Alvarez

15:30 – 15:50 Artificial Intelligence Applications in the Diagnosis of Myeloproliferative Neoplasms

A. Lucchesi

15:50 – 16:10 Building Virtual Cohorts: The Role of Cohort-Based AI Approaches

S. D'Amico

16:10 - 17:30 – THIRD SESSION: The Background for a New Vision on Thalassemia

Chair: S. Rivella

16:10 – 16:30 Cell Cycle Dysregulation in Thalassemia

S. Ghaffari

16:30 – 16:50 Mitophagy and Autophagy in Ineffective Erythropoiesis in β -Thalassemia

D.R. Smith

16:50 – 17:10 Molecules and Pathways Involved in Erythropoiesis: Emerging Therapeutic Targets in Thalassemia

O. Hermine

17:10 – 17:30 Heme-regulated inhibitors as pharmacological inducers of fetal hemoglobin

S. Peslak

17:30 - 18:30 – Roundtable Discussion – International Consensus

Chairs: A. Maggio

Faculties, representatives of patient associations; regulatory agencies (EMA, AIFA); and scientific societies (SIE, SITE, SIIAM, UNIAMO, Fondazione Giambrone), as well as AI and HTA experts and key opinion leaders in rare hematological diseases.

Outcome:

Development of a joint consensus statement and definition of future directions for the establishment of the International GARDEN Consortium on AI-assisted data integration and research in rare hematological diseases.