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

# ***Gene Therapy for Hemoglobinopathies:***

# ***Current Clinical Applications and Future Perspectives***

## *FAD SINCRONA ECM*

### *16th October 2026*

**DATE:** October 16, 2026

**TRAINING HOURS:** 2 hours

**NUMBER OF PARTICIPANTS:** 500

**ECM ID:** 275 - **TBD**

**CME CREDITS GRANTED: 2**

**FAD PLATFORM URL:** https://infomed-ecm.it/

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

**SCIENTIFIC DIRECTOR**
Prof. Aurelio Maggio

**FACULTY:**
Mattia Algeri – Roma (Italy)
Juan Antonio Bueren – Madrid (Spain)
Aurelio Maggio – Palermo (Italy)
Mariasanta Napolitano – Palermo (Italy)

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

**RAZIONALE**

Gene therapy is rapidly reshaping the management of inherited hemoglobin disorders, particularly β-thalassemia and sickle cell disease (SCD). The recent clinical implementation of gene-based therapeutic strategies represents one of the most important advances in hematology and rare diseases, opening new perspectives for potentially curative treatments.

Several gene therapy approaches have now entered clinical practice, demonstrating significant efficacy in reducing transfusion burden, improving quality of life, and modifying disease progression. At the same time, important challenges remain regarding patient selection, access to therapies, long-term safety, sustainability, and integration into healthcare systems.

This ECM webinar aims to provide clinicians, hematologists, pediatricians, transfusion specialists, researchers, and healthcare professionals with an updated overview of the current status of gene therapy for thalassemia and sickle cell disease, with a particular focus on clinical application, patient management, and future therapeutic developments.

The programme will bring together leading Italian experts actively involved in translational research and clinical implementation of advanced therapies for hemoglobinopathies. Particular attention will also be dedicated to emerging next-generation technologies, including gene editing and innovative in vivo therapeutic platforms, which are expected to further expand treatment opportunities in the coming years.

The webinar intends to promote multidisciplinary discussion and strengthen scientific collaboration in the field of advanced therapies for rare hematological diseases

**COURSE TARGET AUDIENCE:**

**Medical Doctor**

- Hematology
- Internal Medicine
- Pediatricians

**Biologist**

- Biologist

**Biomedical Laboratory Technician**

- Biomedical Laboratory Technician

**Nurse**

- Nurse

**OBJECTIVE:**

1. Understand the principles and potential impact of gene editing and innovative therapeutic strategies for hemoglobinopathies
2. Discuss key clinical, organizational and multidisciplinary aspects related to patient selection, treatment pathways and long-term follow-up
3. Describe the current clinical applications of gene therapy in β-thalassemia and other inherited hemoglobinopathies

4. Analyze access, sustainability, registry development and healthcare integration challenges for advanced therapies across different healthcare systems

## SCIENTIFIC PROGRAM

**16:00 – 16:10**
**Opening Remarks and Introduction**
Chair: A. Maggio

***16:10 – 16:35 - FIRST SESSION***
Chair: A. Maggio

**16:10 – 16:35 Gene Therapy for β-Thalassemia: Current Clinical Applications**
M. Algieri

***16:35 – 17:00 - SECOND SESSION***
Chair: M. Algeri

**16:35 – 17:00 Gene Editing and Innovative Strategies for Hemoglobinopathies**
M. Napolitano

***17:00 – 17:25 - THIRD SESSION***
Chair: M. Napolitano

**17:00 – 17:25 Translational Research and Future Therapeutic Platforms in congenital aplastic anemia**
J.A. Bueren

***17:25 – 17:50 Round Table Discussion***
Chair: A. Maggio

Access, Sustainability and Future Perspectives for Advanced Therapies in Italy with the participation of panelists
Discussion topics:

- National organization of gene therapy networks
- Equity of access to advanced therapies
- Long-term follow-up and registries
- Future integration into healthcare systems

**17:50 – 18:00 Conclusions and final remarks**

| COGNOME | NOME | laurea | specializzazione | ENTE DI APPARTENENZA | DESCRIZIONE ATTIVITA' PROFESSIONALE/FORMATIVA |
|---|---|---|---|---|---|
| **Maggio** | Aurelio | Medicina e Chirurgia | Ematologia | Fondazione Franco e Piera Cutino (Palermo) | Presidente Fondazione Cutino e Medico Ematologo (Villa Sofia-Cervello Palermo) |
| **Napolitano** | Mariasanta | Laurea Medicina e Chirurgia | Ematologa | UOC Ematologia e Malattie Rare Campus di Ematologia “FRANCO E PIERA CUTINO” AOR Villa Sofia-Cervello (Palermo) | Direttore UOC Ematologia e Malattie Rare |
| **Algeri** | Mattia | Laurea Magistrale in Medicina e Chirurgia | Oncoematologia | Unità di Trapianto di Cellule Staminali e Terapia Cellulare e Genica dell'IRCCS Ospedale Pediatrico Bambino Gesù (Roma) Università Magna Graecia (Catanzaro) | Dirigente Medico, Ricercatore in Pediatria |
| **Bueren** | Juan Antonio | Laurea in Farmacia | Farmacia | CIEMAT and Biomedical Network Centre for Research on Rare Diseases, CIBERER | Director of the Biomedical Innovation Unit |