



CORSO FAD
FORMAZIONE A DISTANZA SINCRONA



VIRTUAL LIVE EDITION NEW PARADIGMS IN THE MANAGEMENT OF HEMOGLOBINOPATHIES 2021 UPDATE

Thalassemia Day

8th May 2021

ROME TIME (CEST) **13:25 | 18:05**

REGISTER IN THE VIRTUAL PLATFORM:
hemoglobinopathies2021.govirtual.it

Chairpersons:

Aurelio Maggio, Khaled Musallam, Vijay Sankaran

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RATIONALE

In recent years, there has been a swift evolution in development of innovative therapies for the management of thalassemia and sickle cell disease (SCD). Global collaborations have helped transition many of these advances from the bench to the bedside, with gradual integration into routine clinical practice. Conventional therapies have served patients with hemoglobinopathies for decades and significantly improved survival outcomes. Thus, it remains imperative to revisit the holistic care of patients and identify optimal strategies to merge the 'old' with the 'new' in the management of the individual patient. On this International Thalassemia Day, we gather global experts and opinion leaders to discuss various updates in disease understanding and management for patients with hemoglobinopathies. Emerging insights on molecular pathways and clinical forms will be discussed. These would then lead the way to an overview and deep interpretation of novel data from clinical trials of bone marrow transplant, gene therapy, genome editing, and other agents that target the ineffective erythropoiesis and iron dysregulation in thalassemia, as well as sickling, hemolysis and vasculopathy in SCD. We will end the day with sharing experiences on managing patients with hemoglobinopathies during the Covid-19 pandemic.

We value your participation and hope you enjoy the meeting.

Aurelio Maggio

Khaled Musallam

Vijay Sankaran

Acknowledgments

We thank Foundation Franco and Piera Cutino for the support to the organization of the Virtual Congress.

The Campus of Haematology "Franco e Piera Cutino" is a pioneering center for prevention, care and research on Thalassemia and Haemoglobinopathies, and for the reception of patients' families and their guests.

The structure was inaugurated on May 8, 2013 at the Hospital "V. Cervello", A.O.O.R. "Villa Sofia - V. Cervello" of Palermo, under the agreement between the Foundation "Franco e Piera Cutino", the Association for Research "Piera Cutino" Onlus, the U.O.C. Haematology for Rare Diseases of Blood and Haematopoietic Organs and the Health Department of the Regione Sicilia.

The Campus was entirely built thanks to private fundraising by the Association for Research "Piera Cutino", a non-profit organization established in 1998 by the Cutino family.

The Campus of Haematology "Franco e Piera Cutino" stands out as center of excellence across the country, not only for clinical routine tasks but also for the several and exclusive current activities in different clinical and research settings with impact at national and international level.

This is possible thanks to the presence of highly skilled professionals involved in innovative research projects in collaboration with the Foundation "Cutino".

Among the numerous activities carried out at The Campus we are proud to mention

- Coelocentesis: exclusive technique of prenatal diagnosis on fetal cells from Coelomic fluid (the only Center in the world).
- Prenatal diagnosis by Chorionic Villus Sampling and by Amniocentesis.
- Tests to detect Thalassemia healthy carrier.
- First level screening for β -Thalassemia and Haemoglobinopathies.
- Genetic studies for the identification of the CysticFibrosis healthy carrier.
- Biobanking for Research "Anna Maria Ferrara Cutino"
- Outreach and training activities
- Erythroid Cell Culture for the study of HbF drug activation.



A REAL HOPE FOR PATIENTS WITH RARE HAEMATOLOGICAL DISORDERS

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FACULTY

Michael Angastiniotis (Cyprus)

Maria Cappellini (Italy)

Josu De La Fuente (UK)

Giuliana Ferrari (Italy)

Gianluca Forni (Italy)

Baba Inusa (UK)

Mehran Karimi (Iran)

Antonis Kattamis (Greece)

Franco Locatelli (Italy)

Aurelio Maggio (Italy)

Khaled Musallam (UK)

Vijay Sankaran (USA)

Ali Taher (Lebanon)

John Tisdale (USA)

Elliott Vichinsky (USA)

Vip Viprakasit (Thailand)

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SCIENTIFIC PROGRAM

ROME TIME
(CEST)

13.25|13.30

WELCOME TO PARTICIPANTS AND OPENING REMARKS

Aurelio Maggio (Italy), Khaled Musallam (UK), Vijay Sankaran (USA)

SESSION 1

Moderators: Aurelio Maggio (Italy), Antonis Kattamis (Greece)

13.30|13.50

MODERN UNDERSTANDING OF CLINICAL FORMS AND DETERMINANTS OF OUTCOMES IN THALASSEMIA

- Conventional and new classifications
 - The role of anemia/transfusions, iron overload/chelation in shaping disease prospect
- Ali Taher (Lebanon)

13.50|14.10

PATHOPHYSIOLOGY AND CLINICAL CORRELATES IN SCD: STATUS QUO

- Overview of key novel insights into pathophysiology of SCD
 - Clinical observations in children and adults
- Baba Inusa (UK)

14.10|14.30

THALASSEMIA AND SCD FROM THE EYE OF MOLECULAR PATHOLOGY

- Correlations between genotypes and hematologic/clinical profiles
 - Key discoveries in molecular pathways that reshaped our understanding of disease
- Vijay Sankaran (USA)

14.30|14.45

Q & A - Interactive Discussion on the Topics of the Current Session

Faculty

SESSION 2

Moderators: Giuliana Ferrari (Italy), Vijay Sankaran (USA)

14.45|15.05

BONE MARROW TRANSPLANT IN HEMOGLOBINOPATHIES: WHAT HAVE WE ACHIEVED OVER THE YEARS?

- Review of outcomes data
 - Developments in strategy and technique
- John Tisdale (USA)

15.05|15.25

GENE THERAPY: PROGRESS SO FAR

- Available approaches and protocols
 - Current data and ongoing/future trials
- Franco Locatelli (Italy)

15.25|15.45

GENE EDITING: THE NEW KID ON THE BLOCK

- Available approaches and protocols
 - Current data and ongoing/future trials
- Josu De La Fuente (UK)

15.45|16.05

DECISIONAL ALGORITHM FOR GENE THERAPY: RECOMMENDATION FROM "SITE"

- Clinical profile of the ideal candidate for gene therapy
 - Main recommendations for enrollment
- Gianluca Forni (Italy)

16.05|16.20

Q & A - Interactive Discussion on the Topics of the Current Session

Faculty

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SESSION 3

Moderators: Khaled Musallam (UK), Ali Taher (Lebanon)

16.20 16.40	TARGETING INEFFECTIVE ERYTHROPOIESIS AND IRON DYSREGULATION IN THALASSEMIA: EVOLVING STRATEGIES <ul style="list-style-type: none">• Rationale and pathways• Updates from animal studies and ongoing/future trials of novel agents Vip Viprakasit (Thailand)
16.40 17.00	LUSPATERCEPT: OPTIMAL INTEGRATION INTO CLINICAL PRACTICE <ul style="list-style-type: none">• Overall program direction and recent data• Real-life practical implementation Maria Cappellini (Italy)
17.00 17.20	ADVANCES IN MANAGEMENT OF SCD: AGENTS ON THE HORIZON <ul style="list-style-type: none">• Updates on experience with HU• New drugs in development Elliott Vichinsky (USA)
17.20 17.35	Q & A - Interactive Discussion on the Topics of the Current Session Faculty

SESSION 4

Moderator: Michael Angastiniotis (Cyprus)

17.35 17.55	HEMOGLOBINOPATHIES AND COVID-19: SHOULD WE BE CONCERNED? <ul style="list-style-type: none">• Experience and challenges• Strategies and solutions Mehran Karimi (Iran)
17.55 18.00	Q & A - Interactive Discussion on the Topics of the Current Session Faculty
18.00 18.05	Take Home Message & Concluding Remarks Aurelio Maggio (Italy), Khaled Musallam (UK), Vijay Sankaran (USA) ECM QUESTIONNAIRE

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GO VIRTUAL

The **GoVirtual** platform simulates what generally happens in a real Venue.

All locations show clearly marked signs and real time assistance is provided for the entire duration of the Event. The training sessions will be delivered in a dedicated educational area, separated from promotional activities, and directly connected with the CME platform to obtain CME credits according to the current criteria for virtual live streaming events.

REGISTER IN THE VIRTUAL PLATFORM

Connect to the Virtual Platform: **hemoglobinopathies2021.govirtual.it**

- Click on “*Register to attend and get CME credits*” or “*Register to attend with no CME credits*”
- Upon completion of the registration procedure you will be able to:
 - Access the Platform and view all virtual Event contents
 - Attend all LIVE sessions
 - Participate in interactive activities
 - View and download scientific materials
- You can remotely attend the Event by simply connecting online to the platform from:
 - your PC or tablet (the view from smartphones is not recommended)
 - Adequate internet connection/speed

AREA MULTIMEDIALE/ FAST REGISTRATION

■ CLICK HERE



■ Connect to the following link

hemoglobinopathies2021.govirtual.it

■ Scan the QR CODE to connect



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INFORMAZIONI SCIENTIFICHE (ITALIAN PHYSICIANS ONLY)

CREDITI FORMATIVI - ECM: L'evento è stato accreditato al Ministero della Salute dal Provider Collage S.p.A. (cod. rif. Albo Nazionale Provider 309) rif. ECM n. **309-309-315714** per: **Medico Chirurgo, Biologo, Infermiere, Tecnico Sanitario Laboratorio Biomedico** [Ematologia; Pediatria; Medicina Trasfusionale]

OBIETTIVI FORMATIVI: Linee guida - protocolli - procedure

AREA FORMATIVA: obiettivi formativi di sistema

CREDITI ECM: Sono stati assegnati all'evento n. **6** Crediti Formativi.

Per avere diritto ai crediti formativi ECM è obbligatorio:

- Partecipare almeno al 90% dell'attività formativa dichiarata.
- Appartenere ad una delle discipline per le quali è stato accreditato l'evento
- Svolgere la prova di verifica di apprendimento che è garantita dalla rilevazione delle operazioni registrate dal sistema
- La prova di verifica dovrà essere effettuata entro massimo 3 giorni dal termine del corso.
- Il test può essere ripetuto più volte, sino ad un massimo di 5 tentativi e sarà considerato superato al corretto completamento del 75% dei quesiti proposti

Il provider prevederà specifiche verifiche dell'identità del professionista

PLEASE NOTE

Online Registrations will open on March 29 and can only be made via the following platform: <https://hemoglobinopathies2021.govirtual.it>

Certificate of Attendance will be available for all participants who attend the webinar

Information for foreign delegates in order to require the recognition of CME credits, foreign healthcare professionals can submit to their professional order the useful documentation (such as ID, certificate of attendance, program of the event and dedicated request form).

The professional order will evaluate the event and the possible recognition of the credits.

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Organizing Secretariat and CME Provider

COLLAGE S.p.A.

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