

GARDEN

GLOBAL HEMATOLOGICAL RARE DISEASES ALLIANCE

24TH - 25TH
JANUARY 2025
LIVE EDITION

Scientific Committee:
M. Yassin, A. Maggio, A. Srivastava

Organized by
Prof. Aurelio Maggio





*We thank Foundation Franco and Piera Cutino
for the support to the organization of the Event.*



Distinguished Guests, Esteemed Colleagues,

On behalf of the organizing committee, it is with great pleasure that we welcome you to the inaugural **GARDEN Kick-Off Meeting!**

This gathering marks a pivotal moment in our collective efforts to address the multifaceted challenges posed by rare hematological diseases.

The **GARDEN** initiative, **Global hemAtological Rare DisEases alliaNce**, is founded on the belief that collaboration, innovation, and a global perspective are essential to driving meaningful progress in this field. Over the next two days, we will delve into a wide range of topics, from the diverse manifestations of rare hematological diseases to the transformative potential of artificial intelligence in diagnosis and treatment. Our esteemed speakers, representing a diverse spectrum of expertise, will share their insights and perspectives, fostering a rich exchange of knowledge. We are particularly excited to announce the formation of several international working groups, dedicated to tackling critical issues such as data collection, prognostic scoring using AI, and improving patient outcomes. These working groups will serve as a dynamic platform for ongoing collaboration and knowledge sharing, ensuring that the momentum generated during this meeting continues to propel us forward.

We encourage your active participation in the discussions and brainstorming sessions that will be central to our agenda. Your contributions will be invaluable in shaping the future direction of GARDEN and in realizing our shared vision of a world where every individual affected by a rare hematological disease has access to the best possible care and support.

Once again, welcome!

We look forward to two days of productive discussions, fruitful collaborations, and renewed inspiration in our collective journey to improve the lives of patients with rare hematological diseases.

With warmest regards,

Professor Aurelio Maggio

(On behalf of the GARDEN Organizing Committee)

RATIONALE

Hematological rare diseases include both congenital and acquired blood disorders that rely on Hematopoietic Stem Cells. Their rarity affects scientific knowledge, reduces corporate investment, and complicates diagnosis and treatment, leading to patient frustration and distrust in healthcare systems. Definitions of rare diseases vary widely across countries—for instance, 1 in 2,000 births in Europe, fewer than 200,000 patients in the US, and fewer than 50,000 in Japan. By 2015, there were 296 different definitions across 1,109 organizations, but approximately 300 million people worldwide are carriers of a rare disease, impacting millions annually. In sub-Saharan Africa alone, over half a million babies are born with sickle cell disease (SCD), affecting nearly 8 million people globally. The incidence of specific hematological diseases varies by country, and access to innovative treatments is hampered by challenges in clinical trials, costs, reimbursement mechanisms, and necessary investments. Rare hematological diseases represent a global health challenge that requires an urgent and coordinated response. The current fragmentation of healthcare systems and the inequalities in access to care, particularly evident in low- and middle-income countries, highlight the need for coordinated action on a global scale. By bringing together key figures from the sector—doctors, researchers, policymakers, opinion leaders, and representatives from patient associations—the GARDEN (Global hemAtological Rare DisEases alliaNce) aims to create a collaborative and innovative environment capable of addressing the complex challenges posed by rare hematological diseases, improving patient lives, and promoting equity in global health. The distinctive elements of this network include:

- Promoting global alliance among KOL and Associations working on different rare hematological diseases, sharing concerns and planning solutions
- Promoting global multidisciplinary working groups constituted by expert in different scientific fields and representatives of Associations to foster collaboration and bring diverse perspectives, enriching the projects.
- Promoting educational projects and improving international awareness and advocacy
- Promoting research on AI for implementing collection data and developing prognostic scores
- Using all these elements to make together endorsement on single country regulatory agencies aimed at improving accessibility and sustainability of innovative treatments in rare hematological diseases.

The objective of the Kick-off Meeting is to make the basis to develop all these issues.

At the end of the conference, we will implement a follow-up plan to gather feedback from participants and monitor progress toward the established objectives.

SCIENTIFIC PROGRAM

FRIDAY, 24TH JANUARY 2025

SESSION 1 | INTRODUCTION AND NETWORKING

08.30 - 08.45 Welcome and Opening remarks
Giuseppe Cutino, *Franco and Piera Cutino Foundation*
Institutional representatives

08.45 - 09.00 Introduction and Overview on GARDEN project - **A. Maggio**

09.00 - 10.00 **KEYNOTE SPEECH**
CHAIRPERSONS: **M. Cazzola and D. Rund**
The role of AI in the development of data collection and prognostic factors in rare hematological diseases
P. Robinson, M.G. Della Porta, Y. Huang, S. Revel-Vilk

PANEL DISCUSSION 1

10.00 - 12.45 **PANEL VIOLET** - Congenital and Acquired Bone Marrow Failure
CHAIRPERSONS: **L. Luzzatto and E. Solomou**
DISCUSSANTS: **C. Dufour, E. Solomou, L. Luzzatto, A. Bacigalupo**

PANEL TULIP - Bone Marrow Transplantation and Gene Therapy
CHAIRPERSONS: **E. Angelucci and L. Castagna**
DISCUSSANTS: **A. Srivastava, M. Cicalese, F. Locatelli, J. Delafuente, J.F. Tisdale, J.A. Bueren, K. H. M. Kuo**

PANEL ZINNIA - Autoimmune Disorders of Red Cells and Coagulation Pathway
CHAIRPERSON: **S. Siragusa**
DISCUSSANTS: **R. De Cristofaro, K. Aberrahim**

Q&A SESSION

12.45 - 13.15 CHAIRPERSONS: **C. Dufour and J.F. Tisdale**
INVITED INTERVENTIONS:
Representatives of International Patients Associations, Regulatory Agency Members, Policy makers and others
DISCUSSANTS: **All**

13.15 - 14.00 *Break*

SCIENTIFIC PROGRAM

FRIDAY, 24TH JANUARY 2025

PANEL DISCUSSION 2

14.00 - 16.30

PANEL DAISY - Congenital and Acquired Red Cell Disorders no globin-chain-related

CHAIRPERSONS: **A. Iolascon and M. Cazzola**

DISCUSSANTS: **M. Cazzola, R. Russo, A. Iolascon**

PANEL PEONY - Disorders of Coagulation

CHAIRPERSONS: **C. Hermans and M. Napolitano**

DISCUSSANTS: **M. Napolitano, S. Levy-Mendelovich, M. Pinotti, C. Hermans**

PANEL NINFEA - Rare Hematological Disorders of Metabolism

CHAIRPERSONS: **G. Porto and N. Belmatoug**

DISCUSSANTS: **G. Porto, N. Belmatoug**

PANEL SUNFLOWER - Congenital Immunological Disorders

CHAIRPERSON: **M. P. Cicalese**

DISCUSSANTS: **A. Condino Neto, F. Candotti**

PANEL HYDRANGEA - Rare Hematological Disorders of the Reticuloendothelial System

CHAIRPERSON: **M.G. Della Porta**

DISCUSSANTS: **O. Hermine, D. El Fassi**

Q&A SESSION

16.30 - 17.00

CHAIRPERSON: **A. Condino-Neto**

INVITED INTERVENTIONS: **L. Johnson**

and Representatives of International Patients Associations, Regulatory Members, Policy makers and others

SCIENTIFIC PROGRAM

FRIDAY, 24TH JANUARY 2025

BRAINSTORMING

17.00 - 17.30

CHAIRPERSON: **N. Belmatoug**

Issues to be addressed on today topics:

- 1) How do you think Garden may improve awareness, advocacy and education concerns?
- 2) How do you think the Garden may improve clinical research?
- 3) How may KOL and associations networks improve worldwide accessibility and sustainability to innovative treatments?

DISCUSSANTS: **ALL**

WRAP-UP OF DAY 1

17.30 - 17.45

Key takeaways - **A. Maggio**

SATURDAY, 25TH JANUARY 2025

SESSION 2 |

DEFINING MISSION AND OBJECTIVES

08.30 - 08.40

Introduction to the session - **A. Maggio**

08.40 - 10.00

The voice of the patients

CHAIRPERSONS: **I. Marino and A. Magrelli**

ALL representatives of the Associations share their concerns and propose possible solutions.

PANEL DISCUSSION 3

10.00 - 13.00

PANEL PRIMROSE - Thalassemia

CHAIRPERSONS: **M.D. Cappellini and Y. Aydinok**

DISCUSSANTS: **Y. Aydinok, J. Borg, A. El-Beshlawy, G. Forni, S. Mahjoub, M. Yassin, V. Viprakasit**

PANEL CYCLAMEN - SCD

CHAIRPERSON: **M. Abboud**

DISCUSSANTS: **L. De Franceschi, M. Abboud, H. Ezzat, D. Rees, M. De Montalembert**

SCIENTIFIC PROGRAM

SATURDAY, 25TH JANUARY 2025

Q&A SESSION

13.00 - 13.30

CHAIRPERSONS: **K. Musallam, R. Origa**

INVITED INTERVENTIONS:

Representatives of International Patients Associations, Regulatory Agency Members, Policy makers and others

13.30 - 14.00

Break

BRAINSTORMING

14.00 - 15.00

CHAIRPERSONS: **J. L. Vives- Corrons, and R. Farrugia**

Issues to be addressed on today topics:

- 1) How do you think Garden may improve awareness, advocacy and educational concerns?
- 2) How do you think the Garden may improve clinical research?
- 3) How international KOL and associations alliance may influence worldwide accessibility and sustainability to innovative treatments?

Discussants: **ALL**

PLANNING ACTIVITY 2025 - 2026

15.00 - 16.30

CHAIRPERSONS: **A. Maggio and G. Valentini**

- 1) Planning educational events
- 2) Promoting collaboration on data collection and prognostic scores using AI development
- 3) Planning workgroups involving participants from different scientific fields in the same workgroup to foster collaboration and bring diverse perspectives, enriching the projects.

Discussants: **ALL**

CLOSING THE EVENT

16.30 - 17.00

Present next steps and follow-up

Final greetings - **A. Maggio**

FACULTY

ABBOUD MIGUEL	LEBANON
ABERRAHIM KHELIF	TUNISIA
ANGELUCCI EMANUELE	ITALY
AYDINOK YESIM	TURKEY
BACIGALUPO ANDREA	ITALY
BELMATOUG NADIA	FRANCE
BORG JOSEPH	MALTA
BUEREN JUAN	SPAIN
CANDOTTI FABIO	ITALY
CAPPELLINI MARIA DOMENICA	ITALY
CASTAGNA LUCA	ITALY
CAZZOLA MARIO	ITALY
CICALESE MARIA PIA	ITALY
CONDINO-NETO ANTONIO	BRAZIL
DE CRISTOFARO RAIMONDO	ITALY
DE FRANCESCHI LUCIA	ITALY
DE LA FUENTE JOSU	UK
DE MONTALEMBERT MARIANE	FRANCE
DELLA PORTA MATTEO GIOVANNI	ITALY
DUFOUR CARLO	ITALY
EL FASSI DANIEL	DENMARK
EL-BESHLAWY AMAL	EGYPT
EZZAT HATOON	SAUDI ARABIA
FARRUGIA ROSIENNE	MALTA
FORNI GIANLUCA	ITALY
HERMANS CEDRIC	BELGIUM
HERMINE OLIVIER	DENMARK
HUANG YUFEI	USA
IOLASCON ACHILLE	ITALY

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KUO KEVIN	CANADA
LEVY-MENDELOVICH SARINA	ISRAEL
LOCATELLI FRANCO	ITALY
LUZZATTO LUCIO	ITALY
MAGGIO AURELIO	ITALY
MAGRELLI ARMANDO	ITALY
MAHJOUB SONIA	TUNISIA
MARINO IGNAZIO R.	ITALY
MUSALLAM KHALED	UAE
NAPOLITANO MARIASANTA	ITALY
ORIGA RAFFAELLA	ITALY
PINOTTI MIRKO	ITALY
PORTO GRACA	PORTUGAL
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ROBINSON PETER	GERMANY
RUND DEBORAH	ISRAEL
RUSSO ROBERTA	ITALY
SIRAGUSA SERGIO	ITALY
SOLOMOU ELENA	GREECE
SRIVASTAVA ALOK	INDIA
TISDALE JOHN	USA
VALENTINI GIORGIO	ITALY
VIPRAKASIT VIP	THAILAND
VIVES-CORRONS JOAN- LLUIS	SPAIN
YASSIN MOHAMED	QATAR

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ACKNOWLEDGEMENTS

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GENERAL INFORMATION

PROCEDURE OF WORK:

To ensure the correct conduct of the various sessions, all the speakers will have to strictly adhere to the assigned times. Moderators will be required to respect the start times of each session and the time allocated for each presentation.

OFFICIAL LANGUAGE

The official language of the Meeting will be English.
Simultaneous translation will **not** be provided.

LIVE STREAMING EVENT ONLINE ON:

Friday, 24th January: 08.30 – 17.45

Saturday, 25th January: 08.30 – 17.00

Please note that all times are expressed in local time CET (ROME, ITALY)



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HERE**

Organizing Secretariat



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