



# GARDEN

## GLOBAL HEMATOLOGICAL RARE DISEASES ALLIANCE

**24<sup>TH</sup> - 25<sup>TH</sup>**  
**JANUARY 2025**  
**LIVE EDITION**

Scientific Committee:  
**A. Maggio, A. Srivastava, A. Taher**

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, 24<sup>TH</sup> 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, D. Rund**  
The role of AI in the development of data collection and prognostic factors in rare hematological diseases  
**Y. Huang, M. Della Porta, P. Robinson**

## PANEL DISCUSSION 1

**10.00 - 12.00** **PANEL VIOLET** - Congenital and Acquired Bone Marrow Failure  
CHAIRPERSON: **L. Luzzatto**  
DISCUSSANTS: **C. Dufour, E. Solomou, A. Risitano, A. Bacigalupo**

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

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

## Q&A SESSION

**12.00 - 13.00** CHAIRPERSONS: **C. Dufour, J. F. Tisdale**  
INVITED INTERVENTIONS:  
Representatives of International Patients Associations, Regulatory Agency Members, Policy makers and others

**13.00 - 14.00** *Break*

# SCIENTIFIC PROGRAM

FRIDAY, 24<sup>TH</sup> JANUARY 2025

## PANEL DISCUSSION 2

14.00 - 16.00

**PANEL DAISY** - Congenital and Acquired Red Cell Disorders not Globin Chain-Related

CHAIRPERSON: **A. Iolascon**

DISCUSSANTS: **K. H. M. Kuo, M. Cazzola, A. Iolascon**

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**PANEL PEONY** - Disorders of Coagulation

CHAIRPERSONS: **G. Kenet, M. Napolitano**

DISCUSSANTS: **C. Hermans, M. K. Economou**

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**PANEL NINFEA** - Rare Hematological Disorders of Metabolism

CHAIRPERSON: **N. Belmatoug**

DISCUSSANTS: **G. Porto, S. Revel-Vilk, P. Aguilera**

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**PANEL SUNFLOWER** - Congenital Immunological Disorders

CHAIRPERSON: **M. P. Cicalese**

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

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**PANEL HYDRANGEA** - Rare Hematological Disorders of the Reticuloendothelial System

CHAIRPERSON: **O. Hermine**

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

## Q&A SESSION

16.00 - 17.00

CHAIRPERSON: **A. Condino-Neto**

INVITED INTERVENTIONS:

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

# SCIENTIFIC PROGRAM

FRIDAY, 24<sup>TH</sup> JANUARY 2025

## BRAINSTORMING

17.00 - 17.30

CHAIRPERSONS: **S. Revel-Vilk, G. Kenet**

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, 25<sup>TH</sup> 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, A. Magrelli**

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

## PANEL DISCUSSION 3

10.00 - 12.30

**PANEL PRIMROSE** - Thalassemia

CHAIRPERSONS: **A. Taher, M. D. Cappelini**

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

**PANEL CYCLAMEN** - SCD

CHAIRPERSON: **S. L. Thein**

DISCUSSANTS: **L. De Franceschi, A. Glenthøj, M. Abboud, H. Ezzat, D. Rees, M. De Montalembert, S. Lobitz**

# SCIENTIFIC PROGRAM

SATURDAY, 25<sup>TH</sup> JANUARY 2025

## Q&A SESSION

12.30 - 13.00

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

INVITED INTERVENTIONS:

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

13.00 - 14.00

Break

## BRAINSTORMING

14.00 - 15.00

CHAIRPERSONS: **J. L. Vives-Corróns, R. Farruggia**

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

CHAIRPERSON: **A. Maggio**

- 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**

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<b>PINOTTI MIRKO</b>	ITALY
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<b>RISITANO ANTONIO</b>	ITALY
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<b>YASSIN MOHAMED</b>	QATAR

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# AKNOWLEDGEMENTS

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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, 24<sup>th</sup> January: 08.30 – 17.45

Saturday, 25<sup>th</sup> January: 08.30 – 17.00

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



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Organizing Secretariat



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