



# STEM CELL TRANSPLANTATION IN CHILDHOOD BIOTECHNOLOGICAL ADVANCES, ETHICAL ISSUES AND WORLDWIDE EQUAL ACCESS

*Chairs scientific committee:* **Eliane Gluckman, Franco Locatelli**

**26•28**  
**SEPTEMBER**  
**2019**

ROMA EVENTI FONTANA DI TREVI  
Piazza della Pilotta - Roma



**Joint meeting**

Hospital Bambino Gesù - Centre scientifique de Monaco



## RATIONALE

Allogeneic hematopoietic stem cell transplantation (HSCT) is a curative treatment for pediatric patients with many hematological diseases. The indications for HSCT in children are well defined for acute leukemia, as well for patients with non-malignant disease. HSCT from HLA-identical sibling or unrelated donors, such as new techniques of graft manipulation using haploidentical donor graft, allow high chance of cure for those patients. The field of HSCT is evolving fastly with the possibility to perform targeted immunotherapy with CAR-T cells for acute lymphoblastic leukemia, B-cell lymphomas and solid tumors. More recently, the first results of gene therapy for hereditary diseases, open new way of treatment for patients with thalassemia, sickle cell disease, primary immune-deficiencies and metabolic disorders.

The meeting will address all the relevant aspects of indication and results of HSCT in pediatric population, focusing on new insights on gene therapy for hereditary diseases, as well as on the newest results obtained through approaches of immunotherapy. Ethical and regulatory aspects related to these new therapies will be discussed, as well.

Recognized, outstanding international leaders will deliver the main lectures, but also high-level research talks selected from the best abstracts submitted by attendees will be presented.

## ORGANIZERS

**Eliane Gluckman** PARIS, FRANCE AND MONACO, PRINCIPAUTÉ DE MONACO

**Franco Locatelli** ROME, ITALY

## FACULTY

### **William Arcese**

ROME, ITALY

### **Andrea Bacigalupo**

ROME, ITALY

### **Peter Bader**

FRANKFURT, GERMANY

### **Alessandra Biffi**

PADOVA, ITALY

### **Jean-René Binet**

RENNES, FRANCE

### **Jaap Boelens**

NEW YORK NY, USA

### **Francesca Bonifazi**

BOLOGNA, ITALY

### **Juan Bueren**

MADRID, SPAIN

### **Marina Cavazzana**

PARIS, FRANCE

### **Christian Chabannon**

MARSEILLE, FRANCE

### **Fabio Ciceri**

MILANO, ITALY

### **Selim Corbacioglu**

REGENSBURG, GERMANY

### **Paolo Corradini**

MILANO, ITALY

### **Jean Hugues Dalle**

PARIS, FRANCE

### **Francesco Dazzi**

LONDON, UK

### **Mary Eapen**

MILWAUKEE, WI, USA

### **Herman Einsele**

WURTZBURG, GERMANY

### **Fred Falkenburg**

LEIDEN, THE NETHERLAND

### **Dominique Farge**

PARIS, FRANCE

### **Willem Fibbe**

LEIDEN, THE NETHERLAND

### **Patricia Garcia Gonzalez**

SEATTLE WA, USA

### **Corrado Girmenia**

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DURHAM NC, USA

### **Franco Locatelli**

ROME, ITALY

### **Julie Makani**

DAR-ES-SALAAM, TANZANIA

### **Gerard Michel**

MARSEILLE, FRANCE

### **Lorenzo Moretta**

ROME, ITALY

### **Fabienne Mourou**

MONACO, PRINCIPAUTÉ DE MONACO

### **Gilles Pages**

MONACO, PRINCIPAUTÉ DE MONACO

### **Laura Palazzani**

ROME, ITALY

### **Regis Peffault de Latour**

PARIS, FRANCE

### **Andrea Pession**

BOLOGNA, ITALY

### **Lorenza Putignani**

ROME, ITALY

### **Patrick Rampal**

MONACO, PRINCIPAUTÉ DE MONACO

### **Mariusz Z. Ratajczak**

LOUISVILLE KY, USA

### CARDINAL **Gianfranco Ravasi**

VATICAN CITY STATE

### **Katy Rezvani**

HOUSTON TX, USA

### **Vanderson Rocha**

SAN PAOLO, BRASIL

### **Marco Ruella**

PHILADELPHIA PA, USA

### **Annalisa Ruggeri**

ROME, ITALY

### **Riccardo Saccardi**

FIRENZE, ITALY

### **Elizabeth Shpall**

HOUSTON TX, USA

### **Alexis A Thompson**

CHICAGO IL, USA

### **Marcel R.M. van den Brink**

NEW YORK NY, USA

### **Enrico Velardi**

ROME, ITALY

### **Marco Zecca**

PAVIA, ITALY

12.30 | 14.00

Registration open

14:00 | 14.30

Welcome to participants and opening remarks

CARDINAL **Gianfranco Ravasi** VATICAN CITY STATE**Patrick Rampal** MONACO, PRINCIPAUTÉ DE MONACO**Eliane Gluckman** PARIS, FRANCE AND MONACO, PRINCIPAUTÉ DE MONACO**Franco Locatelli** ROME, ITALY**Guillaume Paris** MONACO, PRINCIPAUTÉ DE MONACO**Mariella Enoc** ROME, ITALY**SESSION 1** - INTRODUCTION: STATE OF THE ART OF HSCT

CHAIRPERSONS:

**Eliane Gluckman** PARIS, FRANCE AND MONACO, PRINCIPAUTÉ DE MONACO**Franco Locatelli** ROME, ITALY

14.30 | 15.00

Opening main lecture: Ethic and Science

CARDINAL **Gianfranco Ravasi** VATICAN CITY STATE

15.00 | 15.30

**Mariusz Z. Ratajczak** LOUISVILLE KY, USA

Stem cell biology, what is new?

15.30 | 16.00

**Marcel R.M. van den Brink** NEW YORK NY, USA

Microbiota and cancer

16.00 | 16.30

Coffee break

**SESSION 2** GENE THERAPY OF STEM CELLS FOR INHERITED AND RARE DISORDERS

CHAIRPERSONS:

**Christian Chabannon** MARSEILLE, FRANCE**Selim Corbacioglu** REGENSBURG, GERMANY

16.30 | 17.00

**Marina Cavazzana** PARIS, FRANCE

Gene therapy and genome editing in 2018

17.00 | 17.30

**Alexis A Thompson** CHICAGO IL, USA

How gene therapy will change the fate of patients with Hbpathies

17.30 | 18.00

**Alessandra Biffi** PADOVA, ITALY

Gene therapy for metabolic disorders

18.00 | 18.30

**Juan Bueren** MADRID, SPAIN

Can gene therapy become a reality in patients with inherited bone marrow failure syndromes?

18.30 | 19.00

Discussion

19.00

End of working sessions

19.15

Welcome cocktail

**Day 2**

FRIDAY 27 SEPTEMBER, 2019

### SESSION 3 IMMUNOTHERAPY APPROACHES IN STEM CELL TRANSPLANTATION

CHAIRPERSONS:

**Francesca Bonifazi** BOLOGNA, ITALY

**Paolo Corradini** MILANO, ITALY

08.10 | 08.30

**Marco Ruella** PHILADELPHIA PA, USA

CAR-T cells for acute leukemia:  
will they substitute allogeneic stem cell transplantations?

08.30 | 08.50

**Peter Bader** FRANKFURT, GERMANY

CAR-T cells treatment of pediatric patients with ALL in Germany

08.50 | 09.10

**Lorenzo Moretta** ROME, ITALY

NK cells: from the bench-side to the application in HLA  
Haploidentical stem cell transplantations

09.10 | 09.30

**Fred Falkenburg** LEIDEN, THE NETHERLAND

Have we been really able to dissect the graft-versus-leukemia  
effect from graft-versus-host disease?

09.30 | 09.50

**Herman Einsele** WURTZBURG, GERMANY

Pathogen-specific adoptive immunotherapy: from the proof-of-  
principles studies to the large scale application

09.50 | 10.10

**Katy Rezvani** HOUSTON TX, USA

Cord blood derived NK-CAR cells

10.10 | 10.30

Coffee break

## **SESSION 4** ETHICAL AND SOCIAL ISSUES IN THE ERA OF ADVANCED BIOTECHNOLOGY AND WORLDWIDE EQUAL ACCESS

CHAIRPERSONS:

**Fabio Ciceri** MILANO, ITALY  
**Andrea Pession** BOLOGNA, ITALY

**10.30 | 10.50**

**Julie Makani** DAR-ES-SALAAM, TANZANIA  
Global SCD Research Network: Strategy for Africa

**10.50 | 11.10**

**Vanderson Rocha** SAN PAOLO, BRASIL  
Implementation of HSCT programs in a rapid growing Country

**11.10 | 11.30**

**Patricia Garcia Gonzalez** SEATTLE WA, USA  
The Max Foundation: Equal access to treatment, example of a  
successful paradigm

**11.30 | 12.00**

Discussion

### **NO CME**

**12.00-14.00**

Lunch Special Session on ethics  
INTERACTION WITH GENERAL PUBLIC, MEDIA AND PATIENTS ORGANIZATIONS

*SIMULTANEOUS TRANSLATION WILL BE PROVIDED/TRADUCTION SIMULTANÉE*

PANEL DISCUSSION:

Ethical and social issues in the era of advanced biotechnology and  
Worldwide Equal access/Aspects éthiques et sociaux à l'ère des  
biotechnologies avancées et de l'accès équitable

PANELISTS:

**Franco Locatelli** ROME, ITALY  
**Eliane Gluckman** PARIS, FRANCE - MONACO, PRINCIPAUTÉ DE MONACO  
**Fabienne Mourou** MONACO, PRINCIPAUTÉ DE MONACO  
**Laura Palazzani** ROME, ITALY  
**Jean-René Binet** RENNES, FRANCE  
**Patricia Garcia Gonzalez** SEATTLE WA, USA  
**Julie Makani** DAR-ES-SALAAM, TANZANIA

INTRODUCTION:

**Franco Locatelli** ROME, ITALY

TOPICS:

### **1. Ethics of cell donation / Ethique du don de cellules**

- **Eliane Gluckman**  
Sources of stem cells/ Sources de cellules souches

- **Jean-René Binet**

Ethics of cells donation/ Conditions éthiques du don de cellules

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## 2. Ethical problems of gene manipulation / Problèmes éthiques posés par les manipulations génétiques

- **Laura Palazzani**

Ethical issue of gene editing / Problèmes éthiques de l'édition du gène

- **Fabienne Mourou**

Ethical issues of gene therapy / Problèmes éthiques liés à la thérapie génique

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## 3. Equal access to new therapies in children in developed and developing countries / Accès équitable des nouvelles thérapies chez l'enfant dans les pays en développement

- **Patricia Garcia Gonzales**

Access to drugs in Africa / Accès aux médicaments en Afrique

- **Julie Makani**

Ethics and Equity: Cell and Gene Therapy in Africa / Ethique et Equité: Thérapie cellulaire et génique en Afrique

## SESSION 5 CURRENT INDICATIONS OF STEM CELL TRANSPLANTATION IN CHILDREN

CHAIRPERSONS:

**Mary Eapen** MILWAUKEE WI, USA

**Marco Zecca** PAVIA, ITALY

14.00 | 14.30

**Eliane Gluckman** PARIS, FRANCE - MONACO, PRINCIPAUTÉ DE MONACO

Transplantation for SCD: when and how?

14.30 | 15.00

**Regis Peffault de Latour** PARIS, FRANCE

HSCT in inherited bone marrow failure syndromes

15.00 | 15.30

**Mary Eapen** MILWAUKEE WI, USA

Alternative donor transplant for bone marrow failure syndromes

15.30 | 16.00

**Annalisa Ruggeri** ROME, ITALY

Haploidentical versus cord blood transplantation in children with acute leukemia

16.00 | 16.30

Coffee Break

## SESSION 6 EMERGING INDICATIONS OF STEM CELL THERAPY

CHAIRPERSONS:

**Jean Hugues Dalle** PARIS, FRANCE

**Riccardo Saccardi** FIRENZE, ITALY

16.30 | 17.00

**Joanne Kurtzberg** DURHAM NC, USA

Stem cell transplantation for autism and cerebral palsy

17.00 | 17.30

**Jaap Boelens** NEW YORK NY, USA

HSCT for inherited metabolic disorders: do we still have reasons for investing on it?

17.30 | 18.00

**Dominique Farge** PARIS, FRANCE

HSCT for autoimmune diseases: the competition with targeted therapies

18.00 | 18.30

**Gilles Pages** MONACO, PRINCIPAUTÉ DE MONACO

New generation of anti-angiogenic treatment for advanced pediatric medulloblastoma

18.30

End of working sessions

**Day 3**

SATURDAY 28 SEPTEMBER, 2019

## SESSION 7 HLA-HAPLOIDENTICAL HSCT AND EXPANDED CORD BLOOD TRANSPLANTATION

CHAIRPERSONS:

**William Arcese** ROME, ITALY

**Gerard Michel** MARSEILLE, FRANCE

08.30 | 09.00

**Elizabeth Shpall** HOUSTON TX, USA

Will expansion of hematopoietic progenitors revitalize cord blood transplantation in adults?

09.00 | 09.30

**Franco Locatelli** ROME, ITALY

Novel strategies of graft manipulation for HLA haploidentical HSCT in children

09.30 | 10.00

**Andrea Bacigalupo** ROME, ITALY

Unmanipulated HLA haploidentical HSCT



10.00 | 10.30

**Christian Chabannon** MARSEILLE, FRANCE  
Advances in stem cell engineering

10.30 | 11.00

Coffee break

## SESSION 8 WHAT IS NEW IN HSCT AND CELL THERAPY

CHAIRPERSONS:

**Willem Fibbe** LEIDEN, THE NETHERLAND  
**Vanderson Rocha** SAN PAULO, BRAZIL

11.00 | 11.30

**Francesco Dazzi** LONDON, UK  
MSC: a great future behind?

11.30 | 12.00

**Lorenza Putignani** ROME, ITALY  
Microbiota and pediatric disorders: what are we learning?

12.00 | 12.30

**Enrico Velardi** ROME, ITALY  
Strategy to enhance immune reconstitution in HSCT recipients

12.30 | 13.00

**Corrado Girmenia** ROME, ITALY  
What is new in the diagnosis and management of bacterial and fungal infections in allo-HSCT recipients?

13.00 | 13.30

Discussion

13.30 | 13.45

CME questionnaire

13.45 | 14.00

Take Home Message & Concluding Remarks

**Franco Locatelli** ROME, ITALY  
**Eliane Gluckman** PARIS, FRANCE - MONACO, PRINCIPAUTÉ DE MONACO

## LOGISTICS INFORMATION

### CONGRESS VENUE

- Roma Eventi Fontana di Trevi - Piazza della Pilotta - Roma - Italy

Situated in the heart of Rome, a short walk from The Quirinale and the Fontana di Trevi (Trevi Fountain), from which it gets its name, the Roma Eventi - Fontana di Trevi Conference Centre is the ideal venue for any event. Situated in the splendid Neoclassical palace chosen by Pope Benedict XV as the location for the Gregorian University and built by Guido Barluzzi in 1930, the Conference Centre extends over 1500 sqm and consists of 15 meeting rooms able to accommodate over 1000 participants.

Versatility, high quality services, international level standards and great professionalism all make the "ROMA EVENTI - FONTANA DI TREVI" the ideal location for Conferences, Seminars and Exhibitions.

### HOW TO REACH THE VENUE

<http://www.roma-eventi.com/eng/fontana-di-trevi/business-centre-italy.html>

### FROM 'ROME TERMINI STATION'

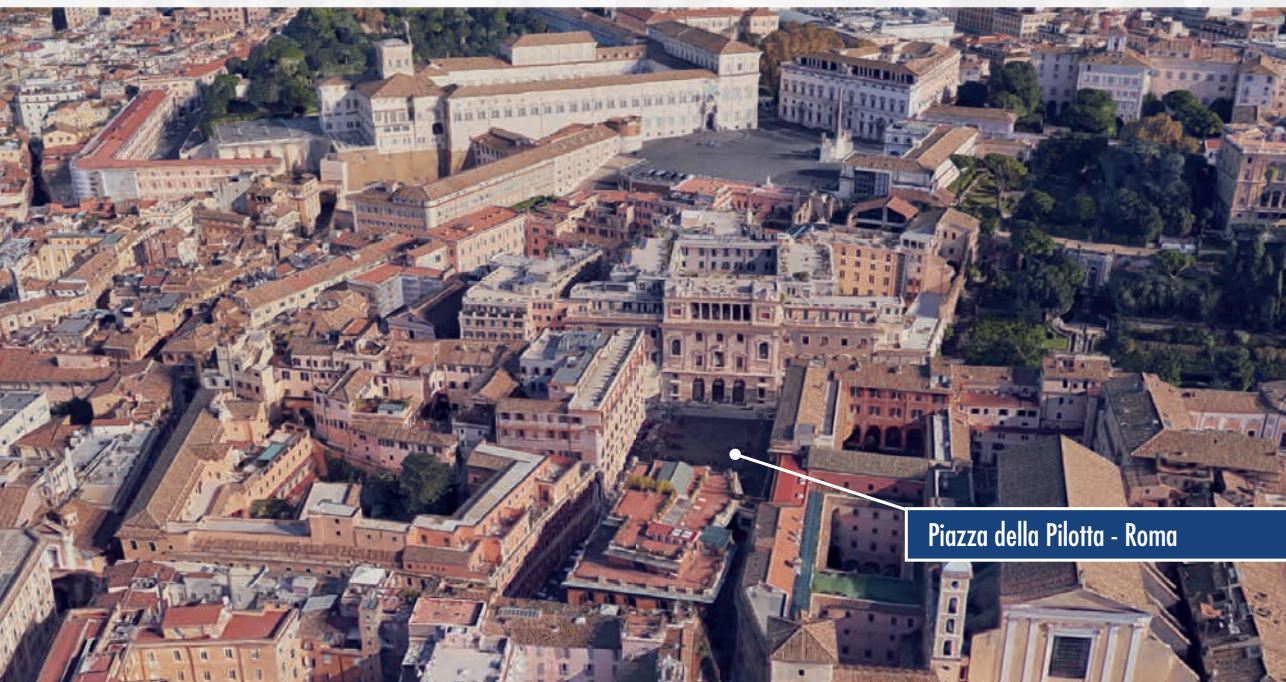
Duration of the Journey: 5 mins (public transport) - 20 mins (on foot)

Distance: 2 km.

### FROM 'LEONARDO DA VINCI' INTERNATIONAL AIRPORT

Duration of the Journey: 40-45 mins (public transport or private car)

Distance: 28 km.



Piazza della Pilotta - Roma

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## SCIENTIFIC PROGRAM

### C.M.E. CONTINUING MEDICAL EDUCATION

Collage S.p.A. (Provider n. 309) has included the Meeting in the Educational Plan 2019.

C.M.E. ref.: 309-258926. The Congress will be suited for **Physicians, Biologists, Medical Lab Technicians (Allergology, Immunology, Hematology, Infectivology, Oncology, Pediatrics, Anesthesiology, Transfusion Medicine, Microbiology, Virology, Biotechnology)**, and provides **4,8 C.M.E. credits**.

In order to obtain C.M.E. credits, participants must:

- attend the 90% of the Meeting (26-27-28 September)
- Fill-in the CME evaluation questionnaire (credits will be granted to those who will answer 75% correctly)
- belong to the credited Medical Profession and Specializations related to this Meeting
- Submit the required documentation, sign in and sign out at the registration desk at the beginning/end of the Congress.

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### C.M.E. CERTIFICATION

The certificate of attendance with the number of C.M.E. assigned credits can be downloaded after 91 days from the date of the Meeting directly through the website [www.collage-spa.it/sezione congressi ecm / corsi ecm](http://www.collage-spa.it/sezione_congressi_ecm/corsi_ecm) / select the year and the title of the Event / digit your fiscal code and download your certification.

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### CERTIFICATE OF ATTENDANCE

Certificate of attendance will be issued at the registration desk at the end of the Meeting.

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### REGISTRATION FEE

It includes: attendance at the scientific sessions, coffee breaks and buffet lunches as indicated in the scientific program, congress badge, congress kit, Social Dinner on September 27, 2019

**Doctors: € 350,00 (22% VAT NOT INCLUDED)**

**Residents & Fellows\*: € 100,00 (22% VAT NOT INCLUDED)**

*\*In order to benefit from the special fee, the delegate is asked to submit an official letter from the institution (PDF format), originally stamped and signed by hand (no electronic signature) by the head of the department confirming this status (the document must be uploaded during the online registration process)*

Please confirm your participation to the Organizing Secretariat by registering online at [www.collage-spa.it / congressi-corsi/](http://www.collage-spa.it/congressi-corsi/) by September 20, 2019.

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### PAYMENTS

PAYMENTS WILL BE POSSIBLE EXCLUSIVELY BY BANK TRANSFER TO COLLAGE S.P.A. ACCOUNT:

IBAN: IT77 V010 0504 6010 0000 0000712

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**Please note:** your registration will only be valid when full payment is received by the administrative office.

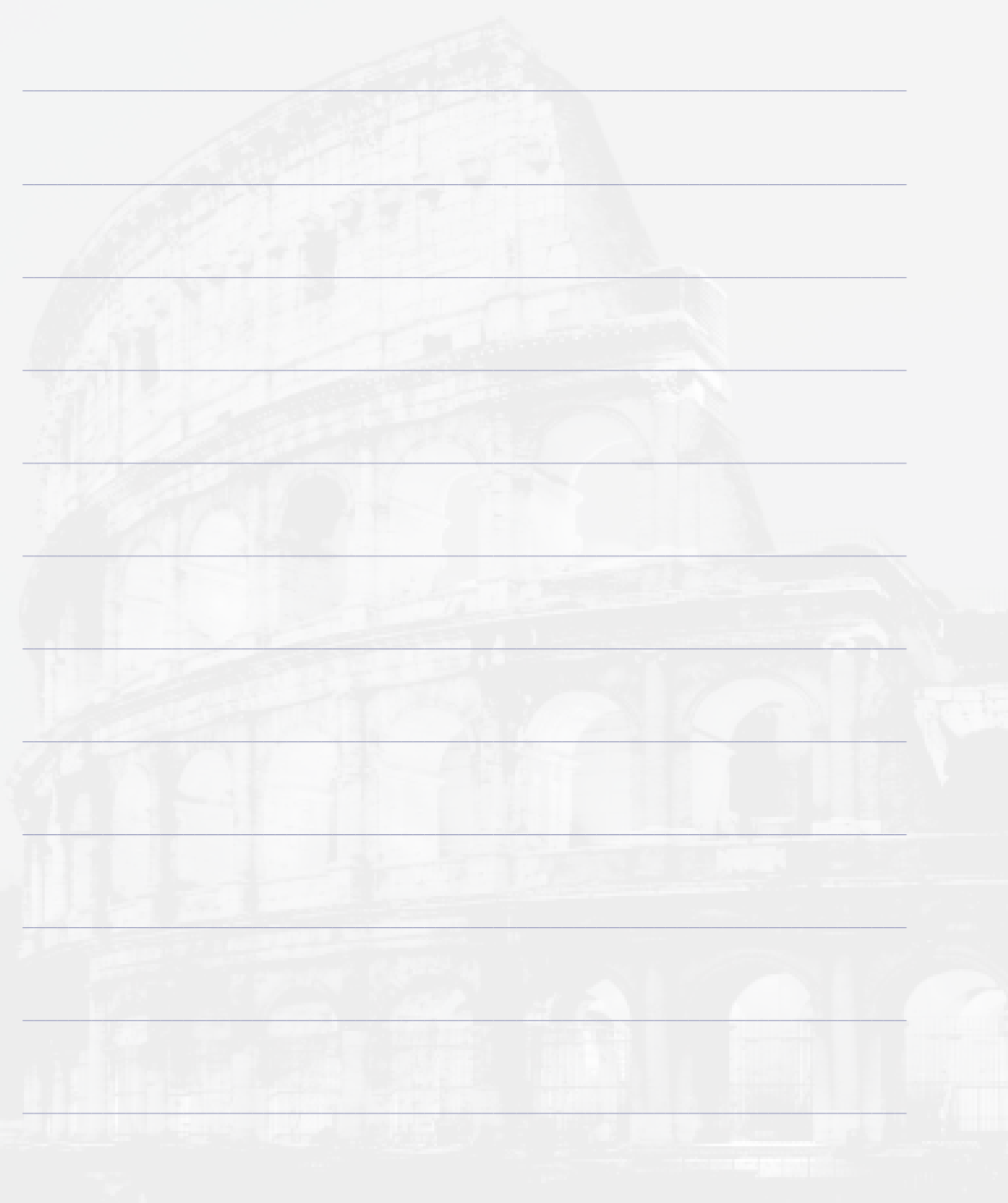
Payment done by bank transfer must be certified with the copy of the receipt of the bank to be uploaded in the registration form or sent via e-mail to [gusy.ventura@collage-spa.it](mailto:gusy.ventura@collage-spa.it)

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### OFFICIAL LANGUAGE

The official language of the Meeting will be **English**.

**NOTES**





Provider & Organizing Secretariat

**COLLAGE** S.p.A.

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