

International Meeting on Childhood MDS and SAA

(EWOG-MDS/SAA)



ROMA EVENTI FONTANA DI TREVI

Piazza della Pilotta - Roma

28•30 SEPTEMBER 2017

President of the meeting: Prof. Franco Locatelli

Scientific committee:

R. De Vito, S. Fenu, K. Girardi, H. Hasle, F. Locatelli, C. Niemeyer, R. Masetti,
P. Merli, J. Stary, B. Strahm, L. Strocchio, M. van den Heuvel

RATIONALE

Childhood myelodysplastic syndromes (MDS) represent a heterogeneous group of rare hematologic disorders (5% of all hematopoietic neoplasms of pediatric age), with distinct subtypes defined by cytogenetics, number of affected lineages, severity of cytopenia, cellular dysplastic morphology, and blast counts.

Underlying genetic predisposition is recognized with increasing frequency in these patients. Extensive next-generation genetic characterization has been recently investigated in childhood MDS, allowing a new diagnostic approach and genetic risk factor stratification. Allogeneic hematopoietic stem cell transplantation (HSCT) offers the potential to cure many patients with MDS. Nowadays, new therapeutic options, including demethylating agents and immunotherapy, are under investigation. In the future, treatment strategy will be tailored based on more refined prognostic factors and molecular findings may help in this setting.

Severe Aplastic Anemia (SAA), the paradigm of bone marrow failure syndrome, is a rare disorder, in which major advancements in therapy have been obtained through the application of immunosuppressive therapy and allogeneic HSCT.

The meeting will address all the relevant biological clinical aspects of childhood MDS and SAA, focusing on new insights on diagnosis, prognosis, and management of these diseases, as well as on the newest results in basic and translational research. 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.

12.30 | 13.30

Registration open

13.30 | 14.00

Opening remarks - [F. Locatelli](#), [M. van den Heuvel](#), [B. Strahm](#)

SESSION I

BIOLOGY OF JMML

CHAIRMEN: [F. Locatelli](#), [J. Stary](#)

14.00 | 14.20

RASopathies - [M. Tartaglia](#)

14.20 | 14.40

The genomic landscape of JMML - [M. Loh](#)

14.40 | 15.00

Epigenetic subclasses in JMML: the development - [D. Lipka](#)

15.00 | 15.20

Epigenetic subclasses in JMML: clinical implications - [C. Flotho](#)

15.20 | 15.40

MicroRNA and JMML - [A. Bertaina](#)

15.40 | 16.00

PDX model in JMML: a strategy for improving both comprehension of pathophysiology and treatment? - [R. Chan](#)

16.00 | 16.30

Discussion

16.30 | 17.00

Coffee break

SESSION II

ORAL SESSION WITH SELECTED COMMUNICATIONS ON JMML AND ADVANCED MDS

CHAIRMEN: [J. Sevilla](#), [S. Bresolin](#)

17.00 | 17.15

Allogeneic hematopoietic stem cell transplantation for therapy-related myelodysplastic syndrome: results of the European Working Group for Myelodysplastic Syndromes (EWOG-MDS) - [R. Bortnick](#)

Thursday - 28 September 2017

- 17.15 | 17.30** Activation of specific transcriptional modules defines maturation states in JMML bone marrow and HSCs - **S. Bresolin**
- 17.30 | 17.45** Whole Genome MBD-seq reveals different CpG methylation patterns in Azacytidine-treated Juvenile Myelomonocytic leukemia (JMML) patients - **P. P. Leoncini**
- 17.45 | 18.00** Integrated molecular profiling of juvenile myelomonocytic leukemia - **N. Murakami**
- 18.00 | 18.15** A Treosulfan-based reduced-toxicity regimen for patients with myelo-dysplastic syndrome with excess of blasts: Results of a EWOG-MDS pilot study - **B. Strahm**
- 18.15 | 18.30** Second allogeneic hematopoietic stem cell transplantation (HSCT) for juvenile myelomonocytic leukaemia (JMML) - **A. Yoshimi**
- 18.30 | 18.45** Discussion on the different presentations of the session - **Faculty**

Friday - 29 September 2017

SESSION III MYELOID MALIGNANCIES PREDISPOSITION SYNDROMES/GENES

CHAIRMEN: B. Dallapiccola, H. Hasle

- 09.00 | 09.25** Overview on familial MDS - **A. Brown**
- 09.25 | 09.45** Genetic aberrations predicting clonal evolution in Fanconi Anemia - **C. Kratz**
- 09.45 | 10.05** Games of clones in congenital neutropenia - **I. P. Touw**
- 10.05 | 10.25** GATA-2 and more in childhood MDS - **M. Wlodarski**

10.25 | 10.45 Discussion on the different presentations of the session - **Faculty**

10.45 | 11.15 Coffee break

SESSION IV

ORAL SESSION WITH SELECTED COMMUNICATIONS ON CHILDHOOD LOW GRADE MDS and SAA

CHAIRMEN: R. Masetti, A. Yoshimi

11.15 | 11.30 Targeted Next-generation-Sequencing (NGS) combined with deep phenotyping for diagnosis of suspected MDS in children - **E. Louka**

11.30 | 11.45 TCRαβ/CD19-cell depleted HLA-haploidentical allogeneic hematopoietic stem cell transplantation (haplo-HSCT): an effective option for childhood myelodysplastic syndromes (MDS)? - **P. Merli**

11.45 | 12.00 Puma-mediated apoptosis of hematopoietic stem cells is a major driver of bone marrow failure in dyskeratosis congenita - **C. Molnár**

12.00 | 12.15 Lymphocyte subsets analysis in peripheral blood shows specific pattern in distinct subgroups of pediatric patients with bone marrow failure - **M. Novakova**

12.15 | 12.30 SAMD9L-Related Familial Myelodysplastic Syndrome and Transient Monosomy - 7 - **S. Sahoo**

12.30 | 12.45 EWOG-MDS study SCT RC RIC 06: Reduced intensity conditioning for children and adolescents with refractory cytopenia of childhood - **B. Strahm**

12.45 | 13.00 Diamond-Blackfan Anemia Phenotype caused by deficiency of Adenosine Deaminase - 2. - **A. Szvetnik**

13.00 | 14.00 Light Lunch

SESSION V

APLASTIC ANEMIA: BIOLOGY AND THERAPY

CHAIRMEN: O. Smith, B. Strahm

- 14.00 | 14.25 Aplastic anemia and clonal evolution: risk factors - [S. Ogawa](#)
- 14.25 | 14.45 PNH and aplastic anemia: friend or foe? - [A. Risitano](#)
- 14.45 | 15.05 Telomeropathies - [I. Dokal](#)
- 15.05 | 15.30 Eltrombopag for low-risk MDS and SAA - [N. Young](#)
- 15.30 | 15.45 Discussion on the different presentations of the session - [Faculty](#)
- 15.45 | 16.15 Coffee break

SESSION VI

ADVANCES IN DIAGNOSIS OF MDS

CHAIRMEN: R. De Vito, M. Schmugge

- 16.15 | 16.35 Does morphology still have a role in the diagnosis of MDS? - [L. Saft](#)
- 16.35 | 16.55 The role of flow-cytometry in the diagnosis of MDS - [A. Orfao](#)
- 16.55 | 17.15 Why I support WHO Classification of childhood MDS - [I. Baumann](#)
- 17.15 | 17.35 What the WHO Classification of adult MDS can teach to pediatricians
[M. Della Porta](#)
- 17.35 | 18.00 Discussion on the different presentations of the session - [Faculty](#)

SESSION VII

CYTOGENETIC/MOLECULAR DIAGNOSIS AND BIOLOGY OF MDS

CHAIRMEN: F. Pasquali, M. Dworzack

- 08.30 | 08.50** What is new in cytogenetics - **C. Mecucci**
- 08.50 | 09.10** What NGS can offer in the diagnosis of childhood MDS - **J. Maciejewsky**
- 09.10 | 09.30** How I can avoid misdiagnosis of inherited BMF syndrome - **A. Shimamura**
- 09.30 | 09.50** The role of microenvironment in MDS - **W. E. Fibbe**
- 09.50 | 10.15** Discussion on the different presentations of the session - **Faculty**
- 10.15 | 10.30** Coffee break

SESSION VIII

TREATMENT OF MDS AND JMML

CHAIRMEN: B. de Moerloose, F. Fagioli

- 10.30 | 10.50** 5-Azacitidine for treatment of patients with MDS and JMML
M. van den Heuvel
- 10.50 | 11.10** How I prevent and treat fungal infections in MDS - **C. Girmenia**
- 11.10 | 11.30** Tailoring the concept of HSCT in JMML in times of 5-azacitidine and genetic profiling - **C. Niemeyer**
- 11.30 | 11.50** How I Treat MDS and AML in Fanconi Anemia - **R. Peffault de Latour**
- 11.50 | 12.10** How I diagnose and treat therapy-related AML - **M. T. Voso**
- 12.10 | 12.30** CAR T Cell Immunotherapy for Myeloid Malignancies - **C. Quintarelli**
- 12.30 | 13.00** Discussion on the different presentations of the session - **Faculty**
- 13.00 | 13.15** CME questionnaire
- 13.15 | 13.30** Closing remarks - **F. Locatelli, M. van den Heuvel, B. Strahm**

Faculty

Irith Baumann

Alice Bertaina

Rachel Bortnick

Silvia Bresolin

Anna Brown

Rebecca Chan

Bruno Dallapiccola

Barbara De Moerloose

Rita De Vito

Matteo Della Porta

Inderjeet Dokal

Michael Dworzack

Franca Fagioli

Wim E. Fibbe

Christian Flotho

Corrado Girmenia

Henrik Hasle

Christian Kratz

Pier Paolo Leoncini

Daniel Lipka

Franco Locatelli

Mignon Loh

Eleni Louka

Jaroslav Maciejewski

Riccardo Masetti

Cristina Mecucci

Pietro Merli

Christian Molnár

Norihiro Murakami

Charlotte Niemeyer

Michaela Novakova

Seishi Ogawa

Alberto Orfao

Francesco Pasquali

Regis Peffault De Latour

Concetta Quintarelli

Antonio Maria Risitano

Leonie Saft

Sushree Sahoo

Marcus Schmugge

Julian Sevilla

Akiko Shimamura

Owen Smith

Jan Stary

Brigitte Strahm

Amina Szvetnik

Marco Tartaglia

Ivo P. Touw

Marry van den Heuvel

Maria Teresa Voso

Marcin Wlordaski

Ayami Yoshimi

Neal Young

CONGRESS VENUE • Roma Eventi Fontana di Trevi - Piazza della Pilotta - Roma

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

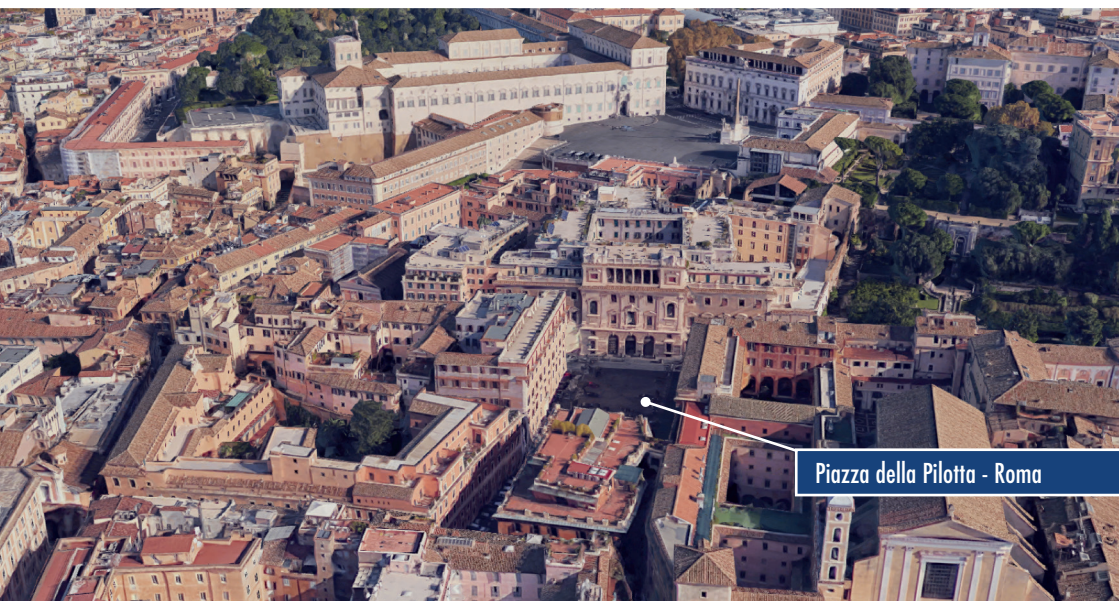
<http://www.roma-eventi.com/eng/fontana-di-trevi/business-centre-italy.html>

From 'ROME TERMINI STATION'

Duration of the Journey: 5 mins (public transport) - 20 mins (on foot) Distance: 2 km.

From the '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 Information

C.M.E. CONTINUING MEDICAL EDUCATION

Collage S.p.A. (Provider n. 309) has included the Meeting in the Educational Plan 2017.

C.M.E. ref.: 309-198192. The Congress will be suited for **Physicians (Hematologists, Oncologists, Pediatricians, Biotechnologists), Biologists** and provides **10.5 C.M.E. credits**.

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

- attend the 90% of the Meeting (28-29-30 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.

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 / select the year and the title of the Event / digit your fiscal code and download your certification.

CERTIFICATE OF ATTENDANCE

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

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 29, 2017

Delegates: € 400,00 (22% VAT not included)

Students/Trainees*: € 300,00 (22% VAT not included)

** In order to benefit from the special fee, the delegate is asked to submit the status confirmation (approval letter signed by the Head of Department) at the Registration Desk*

Please confirm your participation to the Organizing Secretariat by registering online at www.collage-spa.it/congressi-corsi/ by July 25, 2017. **(extended deadline to August 31)**

CALL FOR ABSTRACTS

All abstract submissions must be made electronically through Collage's online abstract submission system.

- The site opens on May 25, and the abstract submission **deadline is June 26, 2017**, 16:00 (GMT).(extended deadline to July 5)
- A Scientific Committee will score the contributions and the Authors will be notified about the abstract acceptance by July 14.
- The best accepted abstracts will be presented during the oral sessions of the meeting (10 minutes for presentation plus 2 for discussion), while the other accepted contributions will be presented as posters.

Please note: All presenters must register at the time of confirming their acceptance of the contribution and pay the conference registration fee.

Please visit www.collage-spa.it/congressi-corsi/ for abstracts guidelines and submission.

PAYMENTS

Payments will be possible exclusively by bank transfer to Collage S.p.A. account:

IBAN: IT77 V010 0504 6010 0000 0000712

BIC: BNL I I TRR

Please note: That 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 to e-mail to giusy.ventura@collage-spa.it

OFFICIAL LANGUAGE

The official language of the Meeting will be English. No simultaneous translation will be provided.

Provider & Organizing Secretariat

COLLAGE S.p.A.

Via U. Giordano, 37/A - 90144 Palermo (Italy) - Tel. +39 091 6867.401 - Fax +39 091 9889354
e-mail: giusy.ventura@collage-spa.it - web: www.collage-spa.it