

inhibitors II

2nd INTERNATIONAL CONFERENCE ON
INHIBITORS IN COAGULATION DISORDERS

MILAN - ITALY. 16 - 17 March 2018

PROGRAMME

SCIENTIFIC COMMITTEE:

F. Peyvandi (ITALY) S. Lacroix-Desmazes (FRANCE)
D. Lillicrap (CANADA) J. Voorberg (THE NETHERLANDS)

ORGANIZED BY



Fondazione IRCCS Ca' Granda
Ospedale Maggiore Policlinico

Sistema Socio Sanitario



Regione
Lombardia



**CENTRO EMOFILIA E TROMBOSI
ANGELO BIANCHI BONOMI**

Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico



UNIVERSITÀ DEGLI STUDI DI MILANO

CONFERENCE VENUES

Friday 16th March

University of Milan

Aula Magna

Via Festa del Perdono, 7
20122 Milano

Saturday 17th March

Centro Pastorale Ambrosiano

Salone Pio XII

Via S. Antonio, 5
20122 Milano

CME ACCREDITATION

The Conference has been accredited by the European Accreditation Council for Continuing Medical Education (EACCME) with **8 European CME credits** (ECMECs). LEE18-00029

16th March 2018: **6,00** credits

17th March 2018: **2,00** credits

European CME Provider: SMC Media srl

To claim your credits please read the instructions at:

www.smc-media.eu/inhibitors2018

ON-SITE SECRETARIAT IS OPEN AT

Friday 16th March

08:00 - 19:00

Saturday 17th March

08:00 - 13:00

Staff may be available beyond these hours and dates for transfer coordination.



ORGANIZING SECRETARIAT: SMC Media Srl

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The 2nd International Conference of Inhibitors on Hemophilia and Coagulation Disorders focuses on the most novel advances in basic science and clinical research for inhibitor development prevention and control at a time when new products and approaches are emerging on the international scene. Their complete understanding is important to **both physicians and researchers** to update our strategies towards haemophilia treatment.

In addition to the educational scope, the conference aims at showcasing the **newest findings** from the industry as well as from independent research groups. The best findings of independent researchers who have spontaneously submitted their abstracts will be featured as oral communications during a special **“Walking Session”** in the foyer in front of the plenary hall.

The 2nd Inhibitor Conference is meant to get science moving, starting from the Walking Session on the first meeting day at the University of Milan. The meeting will then move to Centro Pastorale Ambrosiano on the second day. Social Events will also be *en route*. Hence, an informal and dynamic occasion in which sharing science is at the core of all events.



FACULTY

Donna Di Michele

 USA

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Steven Kitchen

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Christoph Königs

 GERMANY

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Jan Voorberg

 THE NETHERLANDS

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Francesca Stufano

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PROGRAMME

// FRIDAY

16th March 2018

University of Milan, Via Festa del Perdono 7 - Milan
Aula Magna

09:00

SESSION 1: New discoveries on the mechanism of inhibitor development

Chairs: Pier Mannuccio Mannucci (Italy), Donna Di Michele (USA)

Roadmap for improved haemophilia treatment.

Flora Peyvandi (Italy)

Speed bumps on the innovation highway: opportunities to deliberate the course of progress

Donna Di Michele (USA)

Unexpected guests; Stabilin-2 as a regulatory factor for inhibitor development

David Lillcrap (Canada)

IDO1 and inhibitor development in hemophilia A

Davide Matino (Italy)

10:40

Coffee break & Poster Session ([view page](#))

11:00

SESSION 1 (continued)

Chairs: Jan Voorberg (The Netherlands), Christoph Königs (Germany)

Quantification and phenotyping of factor VIII-specific T cells in healthy donors

Bernard Maillère (France)

Antigen presentation in inhibitor development: new discoveries

Sébastien Lacroix-Desmazes (France)

To serve and protect: modulating effect of von Willebrand factor on FVIII peptide presentation

Jan Voorberg (The Netherlands)

12:30

ORAL COMMUNICATION SESSION

Industry research

Chairs: *David Lillicrap (Canada), Elena Santagostino (Italy)*

Emicizumab prophylaxis in adolescent/adult patients with haemophilia A previously receiving episodic or prophylactic bypassing agent treatment: updated analyses from the HAVEN 1 Study

Maria Elisa Mancuso (Italy)

Role of rFVIII Fc in immune tolerance induction in severe hemophilia A patients with inhibitors

Nisha Jain (USA)

Efficacy, safety, and pharmacokinetics of once-weekly emicizumab prophylaxis in paediatric persons with haemophilia A (PwHA) with inhibitors two years old or younger in the HAVEN 2 Study

Flora Peyvandi (Italy)

Low immunogenicity of human-cell-derived recombinant FVIII (simoctocog alfa) in previously untreated patients with non-null F8 mutations

Anna Pavlova (Germany)

Fitusiran, an investigational RNAi therapeutic targeting antithrombin for the treatment of haemophilia

Torben Colberg (USA)

Real-world safety and benefit of early treatment with room temperature-stable recombinant activated factor VII (rFVIIa) in patients with haemophilia A or B with inhibitors: SMART-7™ study results

Kaan Kavakli (Turkey), on behalf of the SMART-7™ steering committee

13:30

Lunch

14:30

SESSION 2:

New developments for tolerance induction

Chairs: *Flora Peyvandi (Italy), Federico Mingozzi (France)*

Gene therapy and new approaches for tolerance induction

Federico Mingozzi (France)

Liver induced tolerance

Percy Knolle (Germany)

15:30

Coffee break & Poster Session ([view page](#))

16:00

SESSION 2 (continued)

Chairs: *Luigi Naldini (Italy), David Lillicrap (Canada)*

Engineering regulatory T cells: Driving CARs to BARs

David Scott (USA)

Perspectives of Immune Tolerance Induction (ITI) in the era of new haemophilia treatment options

Johannes Oldenburg (Germany)

Alternatives to human VIII – orthologs, ancestors and hybrids thereof

Christopher B. Doering (USA)

17:30

WALKING ORAL COMMUNICATIONS SESSION

Foyer in front of the Plenary Hall (with refreshments)

Chairs: *Donna Di Michele (USA), Frits Rosendaal (The Netherlands)*

Generation and characterization of humanized hemophilic mice expressing HLA-DRB1*0101

Jules Russick (France)

Recombinant FVIII product type and the risk of inhibitor development in previously treated patients with hemophilia A: a systematic review and meta-analysis

Shermarke Hassan (The Netherlands)

SIPPET effect on Turkish previously untreated patients (PUP) with severe hemophilia A: national data

Kaan Kavakli (Turkey)

Identification and functional characterization of a novel consensus sequence splicing mutation of factor VIII (FVIII) coagulation gene associated with severe hemophilia A and inhibitors development

Rosella Famà (Italy)

Single center experience in the treatment of haemophilia A PUPs

Susan Halimeh (Germany)

Lot-matching decreases the time for Immune Tolerance Induction (ITI): Preliminary Results of the ObsITI Study

Jonathan Ducore (USA)

Severe acquired von Willebrand syndrome (type 3-like): a case report

Francesca Stufano (Italy)

Discussion and questions and answers

19:00

End of sessions



PROGRAMME

// POSTER SESSION FRIDAY

16th March 2018

From 10:30 and from 15:30 in the coffee break area

FVIII and FIX inhibitors in people living with hemophilia in Cameroon, Africa: a preliminary study

Marie Pauline Ngo Balôgôg Biboum (Cameroon)

Immune tolerance in patients with hemophilia A and inhibitors: preliminary results of the Brazilian Immune Tolerance (BrazIT) Study

Ricardo Camelo (Brazil)

INITIATE (Individualized ITI based on FVIII protection by Vwf): a prospective, randomized, double-blind trial of lot-matched vs random-lot factor VIII (FVIII) concentrate for ITI

Jonathan Ducore (USA)

FVIII expression under the control of its native promoter for hemophilia A gene and cell therapy

Rosella Famà (Italy)

Case study of 22-year-old man with severe hemophilia A and long-standing inhibitor

Edward Laane (Estonia)

The sudden appearance of inhibitors in a severe haemophilia B previously treated patient after the switch to albutrepenonacog alpha

Samantha Pasca (Italy)

Acquired haemophilia at Chris Hani Baragwanath Academic Hospital

Moosa Patel (South Africa)

Thrombocytopenia in association with an alloantibody in type 3 von Willebrand's disease

Irene Regan (Ireland)

Microparticles estimation as predictive tests for monitoring bypassing agents in haemophilia patients with inhibitors

Puja Soni (India)



PROGRAMME

// SATURDAY

17th March 2018

Centro Pastorale Ambrosiano - Via S. Antonio, 5 - Milan
Salone Pio XII

9:00

Lecture: Evolving inhibitor incidence over time

Frits R. Rosendaal (The Netherlands)

9:30

SESSION 3: Treatment of inhibitors in hemophilia

Chairs: *Sébastien Lacroix-Desmazes (France), Michael Makris (UK)*

Development of SerpinPC as a pan-haemophilia agent

Jim Huntington (UK)

Standard and novel approaches for treatment and prevention of bleeding episodes in patients with inhibitors

Roseline d'OIRON (France)

CARs, BARs, immunomodulation - what's next for tackling inhibitors

Christoph Königs (Germany)

11:00

Coffee break

11:30

SESSION 3 (continued)

Chairs: *Sébastien Lacroix-Desmazes (France), Michael Makris (UK)*

How to measure the efficacy of treatment in patients with inhibitors (Laboratory assays)

Steven Kitchen (UK)

How to manage surgery in inhibitors patients with standard and novel therapies

Maria Elisa Mancuso (Italy)

Conclusive remarks and open discussion

Flora Peyvandi (Italy)

13:00

End of sessions

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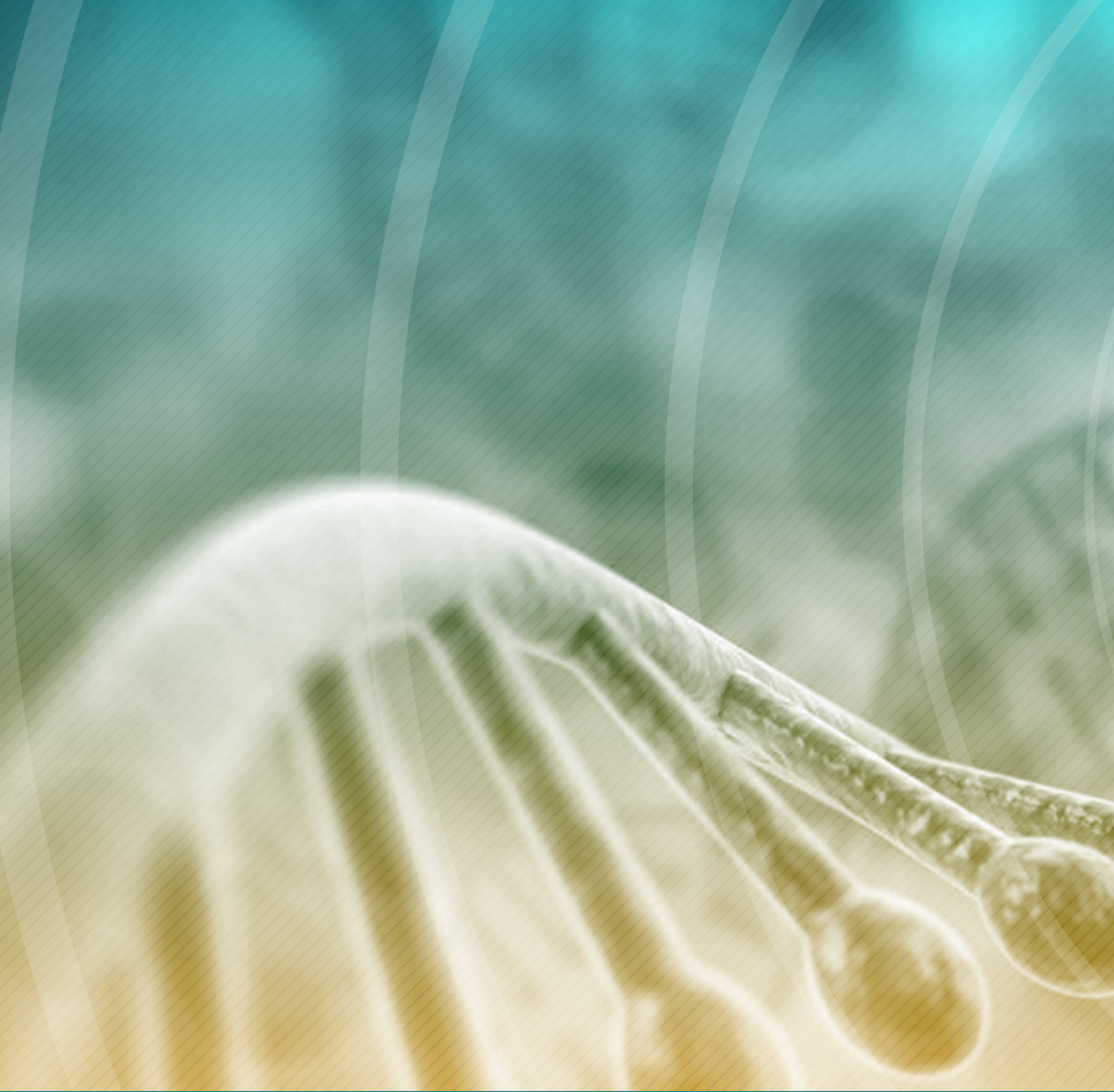


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