

# Paroxysmal Nocturnal Hemoglobinuria:

at the crossroads of somatic mutations,  
clonal expansion and immunity

**Firenze, october 3-4 2024**

**Grand Hotel Baglioni**

## REQUESTED AUSPICES



**Azienda Ospedaliero Universitaria Careggi**



Hematological Diseases  
(ERN EuroBloodNet)



**SIE - Società Italiana di Ematologia**



**October 3, 2024**

10:45-11:00 Welcome and introduction  
**R. Notaro (Firenze),**  
**A.M. Risitano (Avellino)**

### SESSION I

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**Chairmen: R. Notaro (Firenze),**  
**A.M. Risitano (Avellino)**

11:00-12:00 Key note lecture: pathogenesis of PNH  
**L. Luzzatto (Firenze)**

12:00-12:30 Somatic mutation in nonmalignant disease  
**N.S. Young**

12:30-13:00 Complement biology in PNH  
**R.P. Taylor (Charlottesville USA)**

13:00-13:30 PNH and aplastic anemia  
**G. Socié (Paris, France)**

13:30-14:30 *Lunch*

### SESSION II

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**Chairmen: P. Muus (Leeds, UK),**  
**G. Socié (Paris, France)**

14:30-15:00 PNH: a scientific journey from bench to the patient  
**P. Hillmen (Leeds, UK)**

15:00-15:30 C5 inhibition as disease modifying treatment, present and future  
**J. Panse (Aachen, Germany)**

15:30-16:00 Complement inhibition: the never-ending saga  
**A.M. Risitano (Avellino)**

16:00-17:30 **SPONSORED SESSION** (no CME activity)  
**Chairmen: L. Luzzatto (Firenze),  
N.S. Young (Bethesda, USA)**

### 1. SPONSORED LECTURE 1 sponsored by NOVARTIS

Unmet needs in PNH  
**C. Frieri (Avellino)**

### 2. SPONSORED LECTURE 2 sponsored by SOBI

Adding new dimensions to proximal inhibition: transforming paroxysmal nocturnal haemoglobinuria (PNH) management. Broadening horizons with proximal inhibition in PNH  
**A. Kulasekararaj (London, UK)**

### 3. SPONSORED LECTURE 3 sponsored by ALEXION

Proximal complement inhibition without compromising terminal inhibition: 360° approach in PNH  
**S. Sica (Roma)**

17:30-18:15 **PATIENT'S VIEWS** (no CME activity)  
**Chairmen: S. Ferini Strambi (Milano),  
G. Zago (Milano)**

17:30-17:45 Patient reported outcomes in PNH  
**E.N. Oliva (Reggio Calabria)**

17:45-18:00 PNH global alliance  
**M. Piggin (London, UK)**

18:00-18:15 The Max Foundation  
**A. Annamalay (Seattle, USA)**

## October 4, 2024

08:30-08:45 Introduction second day  
**R. Notaro (Firenze),  
A.M. Risitano (Avellino)**

### SESSION III

**Chairmen: C. Gurnari (Roma),  
L. Luzzatto (Firenze)**

08:45-09:15 The biology of clonal hematopoiesis in PNH  
**J.P. Maciejewski (Cleveland, USA)**

09:15-09:45 Latest investigations in PNH  
**C. Nannelli, Firenze**

09:45-10:15 Classical treatment of PNH: when complement inhibitors were (or are) not available  
**H. Schrezenmeier (Hessen, Germany)**

10:15-10:30 *Coffee break*

#### SESSION IV

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**Chairmen: B. Fattizzo (Milano),  
R.P. Taylor (Charlottesville USA)**

10:30-11:00 The biology of complement inhibition in PNH

**C.Q. Schmidt (Ulm, Germany)**

11:00-11:30 C3 binding in PNH patients treated with C5 inhibitors

**R. Notaro (Firenze)**

11:30-12:00 Mechanisms of MAC inhibition by CD59

**D.A. Bubeck (London, UK)**

12:00-13:00 *Lunch*

#### SESSION V: CONTROVERSIES IN PNH

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13:00-13:45 BTH, definition and clinical significance

**Chairman: P. Muus (Leeds, UK)**

**Discussant 1: B. Fattizzo (Milano)**

**Discussant 2: H. Schrezenmeier (Hessen, Germany)**

13:45-14:30 Is the allogeneic stem cell transplantation still an option for PNH therapy?

**Chairman: G. Socié (Paris, France)**

**Discussant 1: C. Frieri (Avellino)**

**Discussant 2: A. Kulasekararaj (London, UK)**

14:30-15:15 Hypoplasia in PNH: to treat or not to treat?

**Chairman: C. Dufour (Genova)**

**Discussant 1: TBA**

**Discussant 2: B. Höchsmann (Ulm, Germany)**

15:15-16:00 Thrombosis: prevention and therapy in anticomplement era

**Chairman: S. Raso (Palermo)**

**Discussant 1: A.P. Iori (Roma)**

**Discussant 2: P. Muus (Leeds, UK)**

16:00-16:45 Terminal or Proximal inhibition?  
Or both is better?

**Chairman: R. Notaro (Firenze)**

**Discussant 1: J. Panse (Aachen, Germany)**

**Discussant 2: A.M. Risitano (Avellino)**

16:45-17:00 CLOSING REMARKS

**R. Notaro (Firenze), A.M. Risitano (Avellino)**



## SCIENTIFIC COMMITTEE

**Antonio Maria Risitano**

AORN Moscati, Avellino

**Rosario Notaro**

Istituto per lo Studio, la Prevenzione  
e la Rete Oncologica (ISPRO), Firenze

## SCIENTIFIC SECRETARIAT

**Maria De Angioletti**

ICCOM, CNR, Firenze

**Gabriella Carlomagno**

Università di Firenze

**Camilla Frieri**

AORN Moscati, Avellino

**Caterina Nannelli**

ISPRO, Firenze

## MEETING VENUE

**Grand Hotel Baglioni**

Piazza dell'Unità Italiana, 6 - 50123 Firenze

Tel. +39 055 23580

[www.hotelbaglioni.it](http://www.hotelbaglioni.it)

## OFFICIAL LANGUAGE

The meeting will be in English with no simultaneous translation into other languages.

## REGISTRATION

Registration is available at [www.ercongressi.it](http://www.ercongressi.it) - section "I nostri congressi/anno 2024".

The registration includes conference kit, participation in the scientific sessions, attendance certificate, coffee break, lunch, slides of the meeting.

## ITALIAN CME CREDITS

An Italian CME application has been submitted as follows:

- Provider Studio ER Congressi SRL: ID 828-426279

- No.11 CME credits for Physician (disciplines: Allergology and Clinical Immunology, Free Choice Pediatric, General Medicine, Hematology, Internal Medicine, Infectious Disease, Medical Genetics Laboratory, Oncology, Pathological Anatomy, Pediatric, Clinical Pathology), Biologist, Pharmacist, Nurse.

- Educational objective: "linee guida-protocolli-procedure"

## ORGANIZING SECRETARIAT

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