

International Workshop Complement system: rare diseases and targeted therapies

Palermo October 13th–14th, 2023 Hotel NH Palermo

Preliminary Program

Focus on the renin-angiotensin, complement, coagulation and kallikrein-kinin system in inflammation: from rare diseases to specific proteins as therapeutic targets.

Rationale

The renin-angiotensin with its pro and anti-inflammatory effects, the complement system with C1 inhibitor that inhibits components of the kallikreinkinin system (KKS), such as activated FXII, FXI and plasma kallikrein, as well plasmin affecting the fibrinolytic system; the coagulation and the KKS, tightly interlinked with the contact system, which is integral to the intrinsic pathway of coagulation (FXI) and thrombus formation; each of them consist of a large number of distinct plasma and membrane bound proteins and receptors that can be activated through proteolytic cascades. Notably, all systems are excessively activated during inflammation, they have potent pro-inflammatory and prothrombotic effects and they increase vascular permeability, leading to edema.

Although the dysregulation of each protein system is involved in the pathogenesis of common disorders, only a few rare diseases such as hereditary angioedema, paroxysmal nocturnal hemoglobinuria, atypical-uremic hemolytic syndrome, C3 glomerulopathies, rare coagulopathies, are approved for clinical treatment targeting renin-angiotensin, complement, coagulation and KKS. The diagnosis and the treatment of these rare diseases are a challenge for health system because it is necessary to improve basic and clinical knowledge and management skills in order to offer the best response to patient's needs. In our workshop we would love to discuss the role of these complex plasma protein systems in disease pathogenesis and then share future pharmacological strategies to treat rare and common diseases with plasma contact factors and complement targeted therapies.

In order to achieve the best results within our workshop, it is of fundamental importance that everyone is an active and responsible part in our job project and in the sharing of final common choices.

Scientific Coordinators

Prof. Francesco Arcoleo

Director of the Unit of Clinical Pathology and Immunology Ospedale Villa Sofia - Cervello, Palermo

Dr. Pietro Accardo

Regional Reference Center Rare Diseases of the Immune System and Angioedema Haemostasis and Thrombosis Center Ospedale Villa Sofia - Cervello, Palermo

Faculty

Pietro Accardo, Palermo Francesco Arcoleo, Palermo Gianluigi Ardissino, Milano Roberta Bulla, Trieste Mauro Cancian, Padova Pierpaolo Coni, Cagliari Davide Firinu, Cagliari Claire Harris, Newcastle upon Tyne (UK) Maurizio Margaglione, Foggia Vincenzo Montinaro, Acquaviva delle Fonti (BA) Paul Morgan, Cardiff (UK) Rosario Notaro, Firenze Santiago Rodriguez de Cordoba, Madrid (ES) Massimo Triggiani, Salerno Andrea Zanichelli, San Donato Milanese (MI)

Friday, October 13th

- 11.00 Registration
- 12.30 Light lunch
- 13.20 Introduction Francesco Arcoleo

1° WORKSHOP Chairman: Andrea Zanichelli

- 13.30 ITACA: a network of Italian centers involved in diagnosis and treatment of rare diseases of kallicrein-kinin system *Mauro Cancian*
- 14.00 The complement system: the overture *Pietro Accardo*
- 14.30 Q&A

2° WORKSHOP Chairman: Maurizio Margaglione

- 14.45 Complement genetics Santiago Rodriguez de Cordoba
- 15.15 The kallicrein-kinin system: genetics and biology *Davide Firinu*
- 15.45 Q&A
- 16.00 Coffee break

Friday, October 13th

3° WORKSHOP Chairman: Vincenzo Montinaro

- 16.15 Complement and neurodegeneration Paul Morgan
- 16.45 Complement and pregnancy *Roberta Bulla*
- 17.15 Complement as target therapy Claire Harris
- 17.45 Q&A Results of all lectures and workshops

18.00 Round table

Pietro Accardo, Francesco Arcoleo, Gianluigi Ardissino, Roberta Bulla, Mauro Cancian, Pierpaolo Coni, Davide Firinu, Claire Harris, Maurizio Margaglione, Vincenzo Montinaro, Paul Morgan, Santiago Rodriguez de Cordoba, Massimo Triggiani, Andrea Zanichelli

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Saturday, October 14th

4° WORKSHOP

Chairman: Santiago Rodriguez de Cordoba

- 09.00 Atypical uremic syndrome *Gianluigi Ardissino*
- 09.30 Parossismal nocturnal hemoglobinuria *Rosario Notaro*
- 10.00 Hereditary angioedema Andrea Zanichelli
- 10.30 C3 glomerulopathy Vincenzo Montinaro
- 11.00 Q&A
- 11.15 Coffee break

5° WORKSHOP Chairman: Paul Morgan

- 11.30 Unmet needs of rare diseases: from patient view to basic and clinical research network *Massimo Triggiani*
- 12.00 Conformational diseases: deficit AAT model *Pierpaolo Coni*
- 12.30 Images of rare diseases: from hereditary angioedema to acquired haemophilia *Pietro Accardo*
- 12.45 Q&A Results of all lectures and workshops

13.00 Round table

Pietro Accardo, Francesco Arcoleo, Gianluigi Ardissino, Roberta Bulla, Mauro Cancian, Pierpaolo Coni, Davide Firinu, Claire Harris, Maurizio Margaglione, Vincenzo Montinaro, Paul Morgan, Santiago Rodriguez de Cordoba, Massimo Triggiani, Andrea Zanichelli

13.15 Light lunch



General information

Official language • English

Venue • NH Hotel Palermo - Foro Italico, 22/B - 90133 Palermo

Registration • Registration is free and includes participation to the workshop, work lunches, coffee breaks, certificate of participation and the CME certificate (to those entitled to). Whomever interested must register on the website *https://www.centercongressi.com/eventi/complementsystem*

CME • The Workshop will be included in the Italian National program of Continuing Medical Education (CME), for the profession of **Psychologist** and **Medical Doctor** with the following specialties: Nephrologists, Molecular Biology, Genetics, Allergology, Internal Medicine, Haematology, Pathology.

To obtain the CME certificate participants must attend 90% of the workshop and correctly answer to 75% of the questions.



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Via G. Quagliariello, 27 • 80131 Napoli • 📞 081.19578490 info@centercongressi.com • www.centercongressi.com