

International Workshop Complement system: rare diseases and targeted therapies

sede: NH HOTEL PALERMO Foro Italico Umberto I, 22/B • 90133 Palermo

Palermo 14-15 novembre 2025

> Chairmen Francesco Arcoleo Pietro Andrea Accardo

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Rationale

The renin-angiotensin system with its pro and anti-inflammatory effects. the complement system with C1 inhibitor that inhibits components of the kallikrein-kinin 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.





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MAIN TOPICS

- THE COMPLEMENT SYSTEM: OUVERTURE
- THE KALLIKREIN-KININ SYSTEM: GENETICS AND BIOLOGY
- THE COMPLEMENT SYSTEM IN HUMAN PATHOLOGY
- COMPLEMENT GENETICS
- COMPLEMENT IN PREGNANCY
- COMPLEMENT AS THERAPEUTIC TARGETS
- ATYPICAL UREMIC HEMOLYTIC SYNDROME
- PAROXYSMAL NOCTURNAL HEMOGLOBINURIA.
- HEREDITARY ANGIOEDEMA
- C3 GLOMERULOPATHY
- UNMEET NEEDS OF RARE DISEASES: FROM PATIENT VIEW TO BASIC AND CLINICAL RESEARCH NETWORK