pediatric rheumatology european society

Macrophage Activation Syndrome/Systemic JIA Working Party

Chair: Claudia Bracaglia (claudia.bracaglia@opbg.net)
Secretary and lead of clinical care: Francesca Minoia (francesca.minoia@policlinico.mi.it)
Lead of science and research: Christoph Kessel (christoph.kessel@uni-muenster.de)
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MACROPHAGE ACTIVATION SYNDROME/SYSTEMIC JIA WORKING PARTY
MADRID 13 June 2019

The 2019 MAS/sJIA WP meeting was attended by 20 participants.

Introduction of the new core of people, elected during the last PReS meeting, according to the new three PReS pillars:

- Chair:
  Claudia Bracaglia, Division of Rheumatology, IRCCS Ospedale Pediatrico Bambino Gesù, Rome, Italy; claudia.bracaglia@opbg.net
- Secretary and lead of clinical care:
  Francesca Minoia, Clinica De Marchi Fondazione IRCCS Ca' Grande Ospedale Maggiore Policlinico, Milan, Italy; francesca.minoia@policlinico.mi.it
- Lead of science and research:
  Christoph Kessel, Department of Pediatric Rheumatology and Immunology, University Hospital Muenster, Germany; christoph.kessel@uni-muenster.de
- Lead of education and training:
  Sebastian Vastert, Pediatric Rheumatology and Immunology, University Medical Center Utrecht, the Netherlands; b.vastert@umcutrecht.nl

The aim of WP is to:
- promote knowledge and international multidisciplinary collaboration among experts in the field of MAS and systemic JIA;
- foster translational research in order to improve care and outcome of patients with MAS and of patients with systemic JIA.

Communications to the group:
- The name of the WP will change in MAS/sJIA WP, as requested to the PReS Council last year.
- Annual calls for projects from the WPs have been founded by PReS/PRINTO collaboration.
  Funds will consist of 10,000/year (total 30,000) in cash provided by PReS, and of an

equivalent amount in manpower for implementation and infrastructure provided by PRINTO. Each PReS/PRINTO grant will be funded for a maximum of 3 years. The proposals will be peer reviewed by members of the PReS Council and members of the Advisory Council of PRINTO.
2019: deadline for submission will be September 30, 2019
2020: deadline for submission will be April 30, 2020
The granted projects and updates will be presented at Annual PReS Meeting in a session dedicated to the research of the WPs.

Presentation of ongoing research projects:

1. ***"Development of new criteria for primary HLH"***. This project aims to refine the diagnostic criteria for familial HLH, based on real patient data, using simple, valuable, reliable and reasonably available diagnostic parameters. AnnaCarin Horne presented the preliminary results of this study. The study is still open because both more cases of FHL and more controls are needed.

2. ***"Validation of risk score for MAS in sJIA patients"*.** The objective of this study is to define a risk score of MAS for sJIA patients using the routine laboratory parameters of disease activity and severity. Claudia Bracaglia presented the initial results of this project. The MAS risk score was defined on a developmental cohort created with 53 sJIA patients, with or without MAS, enrolled from Ospedale Pediatrico Bambino Gesù in Rome. Now the score needs to be validated on a larger population. So far data of 74 patients were collected from other 7 paediatric rheumatologic European centres but only 47 of these patients can be used for the analysis. The study is still open to increase the number of patients in the validation cohort.

3. ***"MAS patients with systemic thrombotic microangiopathy (TMA)"***. The aim of this study is to describe clinical and laboratory features, therapeutic choices and outcome of a multinational cohort of pediatric patients with MAS/sHLH and TMA. Francesca Minoia presented the initial results of this study: a total of 23 patients with MAS/sHLH (18 MAS and 5 sHLH) and TMA were collected so far at 14 centres in 7 countries. The study is still open to enrol patients.

Proposal of new collaborative research projects:

1. ***"ReSyst study***". Bas Vastert and Claudia Bracaglia proposed this international collaborative study, connecting existing large national sJIA cohorts and open for new patients from paediatric rheumatology centres throughout Europe/world. The aim of this study is to collect biosamples and clinical data from patients with a refractory course of their disease.

2. ***"Analysis of type I IFN score and IL-18 expression in MAS and MAS risk patients"***. Christoph Kessel proposed a translations research project which aims to evaluate the possible association between type I IFN signalling and IL-18 gene expression in MAS and MAS risk patients.

Clinical proposal:

1. ***"Proposal for a survey on the currently used treatment for sJIA and for MAS/sHLH in the European Paediatric Rheumatology centres".*** Francesca Minoia and Bas Vastert proposed this clinical project aimed to understand the currently used/available protocols for sJIA, MAS in sJIA vs MAS in other immune mediated diseases and in secondary HLH.
   The current international recommendations for sJIA are limited by clinical evidences at the moment of publication (2013 ACR/EULAR recommendations, 2012 CARRA treatment plans) and there are not randomized trials or widely accepted recommendations for the treatment of MAS/sHLH patients. The aim of this project is to understand the real-life experience in sJIA/MAS and sHLH treated patients in European Paediatric Rheumatologic Centres.