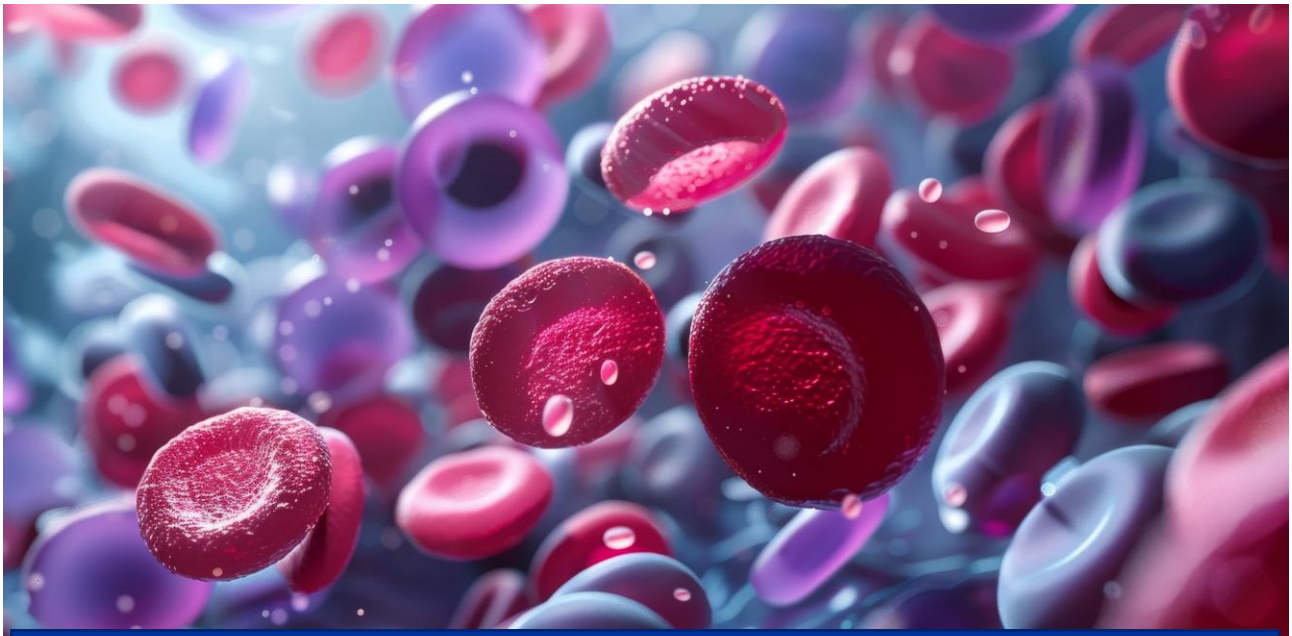
EMA
EUROPEAN MEDICINES AGENCY



Workshop - Challenges in drug development, regulation and clinical practice in immune thrombocytopenia

30th June 2026 (14 pm to 18 pm, CET)

Virtual meeting

Background and Objectives

Primary immune thrombocytopenia (ITP) is an acquired immune mediated disorder characterised by isolated thrombocytopenia, defined as a peripheral blood platelet count less than 100 x 109/L, and the absence of any underlying cause.

Recently, new therapies have been authorised and further medicines are in development.

The clinical requirements to support a marketing authorisation application in EU are laid down in scientific guidelines, namely the "Guideline on the clinical development of medicinal products intended for the treatment of chronic primary immune thrombocytopenia (EMA/CHMP/153191/2013)".

In line with the clinical domain working party work plan for 2026, this EMA workshop is organised to have a multi-stakeholder's perspective on the challenges in drug development, regulation and clinical practice in immune thrombocytopenia.

This workshop is organised to have a multi-stakeholder's perspectives on this disease before initiating an update of the scientific guideline, namely the "Guideline on the clinical development of medicinal products intended for the treatment of chronic primary immune thrombocytopenia (EMA/CHMP/153191/2013)"

The aims of the workshop are:

- To present the epidemiology and disease background in adults and children with immune thrombocytopenia, the current international treatment guidelines, the unmet medical need, and the overview of the authorised medicines/treatments for immune thrombocytopenia.

- To present the challenges in treatment/drug development from a clinicians', patients and regulator´s perspective with regards to study design, objectives and endpoints used in clinical trials.

Practical information:

The workshop can be accessed via Teams with the link provided in the meeting invitation.

Challenges in drug development, regulation and clinical practice in immune thrombocytopenia

Chaired by: Daniela Philadelphy, CHMP member for Austria and Haematology working party chair, EMA

13:30 Joining and technical checks

14:00 Welcome and opening speech

Opening remarks **10'**
Caroline Voltz-Girolt, Office of advanced therapies and haemato-oncology diseases, EMA

Outline of the day and objectives **10'**
Daniela Philadelphy, CHMP member for Austria and Haematology working party chair at EMA

14:20 Session 1: Clinical considerations on immune thrombocytopenia and patient´s perspectives

Diagnosis, epidemiology and patient characteristics of immune thrombocytopenia **15'**
Maria Lozano, Hospital JM Morales Meseguer, Murcia, Spain

Clinical management, treatment goals and treatment guidelines **15'**
Quentin Hill, Leeds Teaching Hospitals, UK

Paediatric perspectives and treatment goals **15'**
Michele P Lambert, Children's Hospital of Philadelphia, USA

Patients' perspective on immune thrombocytopenia **15'**
Barbara Lovrencic – Aipit, Italian Association of Immune Thrombocytopenic Purpura

15:20 Coffee Break

15:40 Session 2: Overview of authorised medicines in immune thrombocytopenia and regulatory considerations

Authorised medicines and regulatory considerations by PMDA **10'**
Ryo Matsuoka, medical officer at office of new drug II, Pharmaceuticals and Medical Devices Agency (PMDA)

Authorised medicines and regulatory considerations by FDA CBER **10'**

Christine Knoll, clinical reviewer at the Benign Hematology Branch at U.S. FDA Center for Biologics Evaluation and Research

Authorised medicines and regulatory considerations by FDA CDER **10'**

Roma Rajput, clinical reviewer and team leader at the U.S. FDA within the Center for Drug Evaluation and Research

Authorised medicines and regulatory considerations by Health Canada **10'**

Koushalya Parmsothy, medical evaluator, clinical evaluation division: oncology and haematology, Health Canada

Authorised medicines and regulatory considerations by EMA **10'**

Edward Laane, CHMP member for Estonia, EMA

16:40 Session 3: Clinical development

Considerations on study populations **15'**

Walter Johannes Beiersdorf, senior expert and clinical assessor at the Austrian Agency for Health and Food Safety (AGES)

Considerations on study objectives and outcome parameters **15'**

Ole Weis Bjerrum, clinical assessor at the Danish Medicines Agency Danish Agency (DKMA)

ERCI-IWG (2026) revised standardization of terminology, definitions, and outcome criteria in ITP in adults and children: a preliminary presentation **15'**

Francesco Rodeghiero, scientific director at hematology project foundation, Vicenza, Italy

17:30 Panel discussion and questions and answers

Chairs: Daniela Philadelphy, CHMP member, Austria and Haematology working party chair
Caroline Voltz, Scientific secretary of the heamatology working party, EMA

Discussion on relevant parameters and outcome measures to be collected in clinical trials by considering treatment goals, patient characteristics and differences in patient populations, study designs aspects for confirmatory trials and specific safety and efficacy considerations.

17:55 Closing remarks

Wrap up **5'**

Daniela Philadelphy