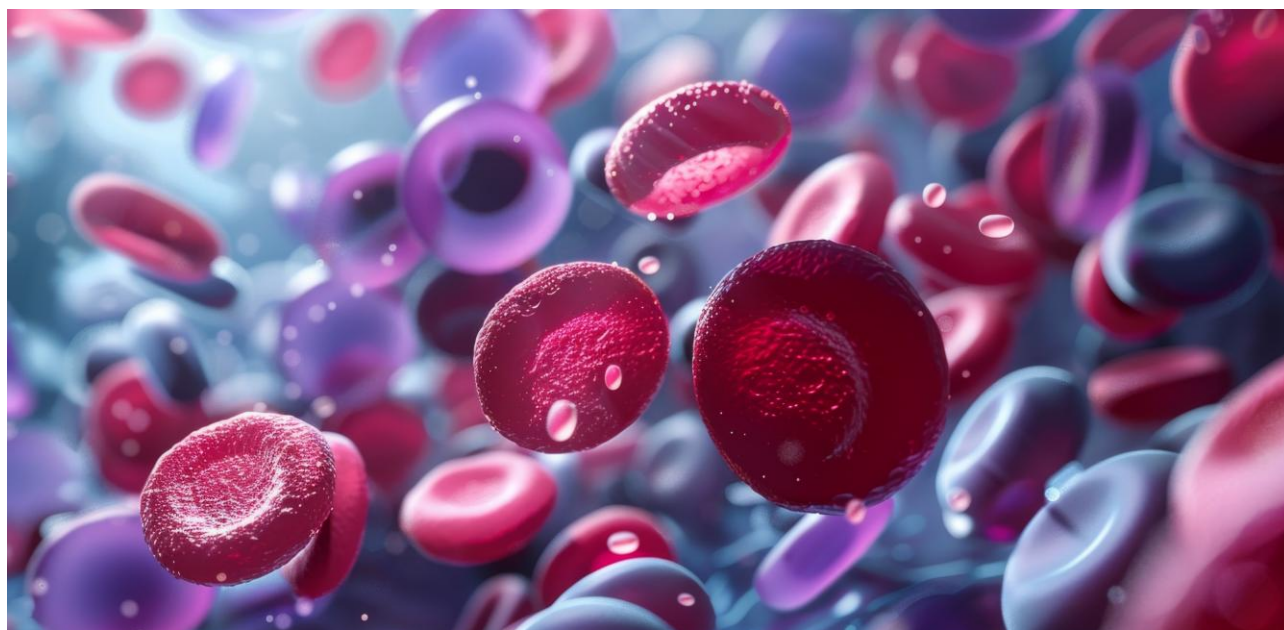




## **Workshop - Challenges in drug development, regulation and clinical practice in immune thrombocytopenia**

30<sup>th</sup> June 2026 (14 pm to 18 pm, CET)

Virtual meeting

### **Background and Objectives**

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Primary immune thrombocytopenia (ITP) is an acquired immune mediated disorder characterised by isolated thrombocytopenia, defined as a peripheral blood platelet count less than  $100 \times 10^9/L$ , 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*

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## **13:30      Joining and technical checks**

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## **14:00      Welcome and opening speech**

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**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*

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

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**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*

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## **15:20      Coffee Break**

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

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**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*

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**16:40 Session 3: Clinical development**

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**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)*

**ERICI-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*

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**17:30 Panel discussion and questions and answers**

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*Chairs: Daniela Philadelphia, CHMP member, Austria and Haematology working party chair*

*Caroline Voltz, Scientific secretary of the haematology working party, EMA*

Discussion on clinical questions of interest to be addressed by clinical trials in the development of ITP medicinal products, relating to study objectives, target population and patient characteristics, outcome variables to be collected and relevant intercurrent events.

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**17:55 Closing remarks**

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**Wrap up 5'**

*Daniela Philadelphia*