EMA
EUROPEAN MEDICINES AGENCY

Workshop - Challenges in drug development, regulation and clinical practice for immunoglobulins

5th March 2025 (14 pm to 18 pm, CET)

Virtual meeting

Background and Objectives

The first use of polyvalent immunoglobulin preparations was as replacement therapy in humoral immunodeficiency situations. As human normal immunoglobulin for intravenous use (IVIg) and for subcutaneous and intramuscular use (SCIg/IMIg) are prepared from plasma collected from a high number of healthy blood and plasma donors, the spectrum of anti- body specificity expressed by the immunoglobulin G (IgG) is large. Among the antibody specificity spectrum, IVIg and SCIg/IMIg recognise many bacterial, viral and other infectious agent antigens, and a large number of self-antigens.

The therapeutic effect in replacement covers primary immunodeficiencies (PID) and several secondary immunodeficiencies (SID). IVIg/SCIg/IMIg have also been used in a clinical setting for their immunomodulatory activity. The immunomodulatory indications for IVIgs based on clinical trials with various IVIg products are primary immune thrombocytopenia (ITP), Guillain-Barré syndrome (GBS), Kawasaki's disease, multifocal motor neuropathy (MMN), and chronic inflammatory demyelinating polyradiculoneuropathy (CIDP). However, contrary to the "established immunomodulatory indications" for IVIgs, SCIgs do not yet have clearly "established" indications in this area, investigations for some auto-immune disorders are on-going and some of the chronic neurological disorders have now become the focus of attention due to possible advantages home treatment would provide.

The clinical requirements to support a marketing authorisation application in EU are laid down in scientific guidelines, namely the "Guideline on the clinical investigation of human normal immunoglobulin for intravenous administration (IVIg) (EMA/CHMP/BPWP/94033/2007 rev. 4) " and the "Guideline on the clinical investigation of human normal immunoglobulin for subcutaneous and/or intramuscular administration (SCIg/IMIg) (EMA/CHMP/BPWP/496692/2023 rev 2)".

In line with the haematology working party work plan for 2025, this workshop is organised to have a multi-stakeholder's perspective on the use of immunoglobulins as the scientific guideline on the clinical investigation of human normal immunoglobulin for subcutaneous and/or intramuscular administration (SCIg/IMIg) and Core SmPC are under public consultation until 31 May 2025. These guidelines are aimed to define the data needed and clinical requirements for benefit-risk evaluation and obtaining marketing authorisation.

The aims of the workshop are:

- To present the current regulatory requirements for the clinical development of immunoglobulins in support of a marketing authorisation application.
- To present the clinicians' / healthcare professionals' perspective on the use of immunoglobulins and their view on established and new indications.
- To present perspectives from industry and Health Technology Assessment bodies on the use of immunoglobulins.

Practical information:

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

Challenges in drug development, regulation and clinical practice for immunoglobulins

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'**
Daniela Philadelphy, CHMP member for Austria and Haematology working party chair

Outline of the day and objectives **5'**
Caroline Voltz-Girolt, Office of advanced therapies and haemato-oncology diseases, EMA

Authorised medicines and regulatory considerations by EMA **15'**
Claudia Gramiccioni, Haematology working party member, AIFA, Italy

14:45 Session 2: Clinical perspectives on the use of immunoglobulins

Clinical considerations on the use of IgG in primary immunodeficiencies (replacement therapy) **10'**
Dr. Maria Pia Cicalese, San Raffaele Scientific Institute and Vita-Salute San Raffaele University, Milan, Italy

Clinical considerations on the use of IgG in secondary immunodeficiencies (replacement therapy) **10'**
Dr. Peter Asdahl, Aarhus University Hospital, Denmark

Clinical considerations on the use of IgG immune thrombocytopenia (immunomodulatory indications) **10'**
Pr. Jaap Jan Zwaginga, chairman of the Benign Hematologic Diseases working party of the Dutch Hematology Association, The Netherlands

Clinical considerations on the use of IgG in chronic inflammatory demyelinating polyneuropathy CIDP (immunomodulatory indications) **10'**
Dr. Yuri Falzone, San Raffaele Scientific Institute, Milan, Italy

Clinical considerations on the use of IgG in neuropathies (immunomodulatory indications) **10'**
Prof. Helmar Lehmann, Director of the Klinik für Neurologie und Geriatrie, Leverkusen, Germany

16:00 Coffee Break

16:15 Session 3: Additional perspectives

PPTA industry perspectives on the clinical development and clinical use **10'**
James R. Knowles, PhD- Senior Director of Global Regulatory Policy at PPTA

EuropaBio perspectives on the clinical development and clinical use **10'**
Vai Katkade, MD, PhD, Senior Director, Medical Affairs at CSL Behring

IPFA perspectives on the clinical development and clinical use **10'**
Karen Pinachyan, Head of Scientific, Medical and Regulatory Affairs, LFB

HTA perspectives **10'**
Anja Schiel, Special Advisor, Norwegian Medicines Agency

Monitoring of supply for immunoglobulins **10'**
Klaus Kruttwig, Medicines and Medical Devices Shortages Specialist, EMA

17:00 Panel discussion

Chairs: Daniela Philadelphy, CHMP member, Austria and Haematology working party chair
Caroline Voltz, EMA

Discussion on the use of immunoglobulins from a regulatory and clinical perspective, the clinical development of immunoglobulins and the need to update and amend the existing EMA clinical guidelines.

17:55 Closing remarks

Wrap up **5'**
Daniela Philadelphy