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Haematology working party
Human Medicines Division

Consolidated 3-year rolling work plan for the Haematology Working Party (HAEMWP)

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Work plan period: January 2025 – December 2027 (with a first review point after one year)

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1. Strategic goals

1.1. Short-term strategic goals

The area of expertise of the Haematology Working Party (HAEMWP) is in non-malignant haematology and includes conditions for which medicinal products derived from human plasma and their recombinant analogues are used.

As such, the HAEMWP covers clinical considerations for manifold biological products (which were traditionally referred to as blood products) manufactured from plasma, such as immunoglobulins, coagulation factors, human albumin and fibrin sealants and used in a range of indications. It also covers any medicinal product intended for haematological indications, such as haemophilia e.g. plasma-derived and their recombinant alternatives (rFVIIa, rFVIII, rFIX, rFX, rFXIII), and novel non-replacement haemophilia therapies. In supporting the CHMP, in the context of scientific evaluations or the development of guidelines, close collaboration with other working parties and committees may be needed, such as for example the Committee for Advanced Therapies (CAT) in case of advanced therapy medicinal products (ATMPs).

Numerous interactions take place with the EC substances of human origin (SOHO) team, the European Center for Disease and Control (ECDC), the European Directorate for Quality of Medicines (EDQM), relevant plasma industry organisations, other stakeholders and learned societies concerning the security of blood and plasma supply in light of increasing demand for plasma products but also concerning new developments in this area. The HAEMWP is also committed to continue its long-standing engagement with patients' organisations in relation to possible shortages of essential plasma products (e.g., immunoglobulins) and to build any new engagements in the field of non-malignant haemotology.

Considering the new developments in the fields of non-haematology, the HAEMWP is starting new guidelines in sickle cell disease and beta-thalassemia following the workshop on hemoglobinopathies held in 2024.

1.2. Long-term strategic goals

- Cooperate with Registry Holders to coordinate data collection and evaluation (this is part of the Regulatory Science Strategy (RSS) 2025).
- Contribute to regulatory research projects.
- Participate to the Joint EMA/Industry Task Force (JEIF) meeting.
- Provide support (as relevant) to help promoting the increase of plasma donation and supply and liaise accordingly with Single Point of Contact (SPOC) working Party, the EC SOHO team and relevant stakeholders, Biologics Working Party (BWP) (e.g. updating the eligibility donor criteria; collaboration concerning Plasma Master File (PMFs)).
- Continuous engagement with plasma industry associations on relevant regulatory and clinical scientific matters.
- Active contribution to the Blood cluster TCs with FDA and Health Canada to discuss specific issues arising from medicines under development or scientific evaluation of products, global plasma issues as well as guidelines with the aim of exchanging information and achieving where possible regulatory convergence in relation to the clinical development, surveillance and

regulatory actions (safety issues) of medicines intended for non-malignant haematology conditions.

- Active contributions to ad hoc TC with CBER and CDER in the non-malignant heamatology area.
- Active engagement with learned societies and relevant patients' organisations, via participation to events to discuss relevant issues or promote guidelines.
- Cooperation with the EC SOHO team, EDQM and ECDC.
- Outreach activities via publication of summary of product evaluation or areas of interest, according to the publication plan agreed by HAEMWP.
- Enhancement of haematology network capability and expertise building by developing haematology European Specialised Expert Community (ESEC) disease specific webinars.
- Multidisciplinary collaboration: Given the broad spectrum of indications for immunoglobulins and their possible further expansion into other medical areas, also the importance of quality-related aspects for clinical evaluation and the nature of products, interaction with other committees/WPs is needed (e.g. BWP, Methodology Working Party, the Paediatric Committee (PDCO), CAT, the Pharmacovigilance Committee (PRAC), the Orphan Committee (COMP), the Clinical domain WPs as relevant).

2. Tactical goals

2.1. Guidance activities

(A) Activities ongoing/to be finalised in 2025

Action: Lead

Clinical investigation of human normal immunoglobulin for subcutaneous and/or intramuscular administration (SCIg/IMIg) (CHMP/BPWP/410415/2011 Rev. 1) and core summary of product characteristics for human normal immunoglobulin for subcutaneous and/or intramuscular administration (EMA/CHMP/BPWP/143744/2011 rev. 1)

Target date Finalise guideline following public consultation Q3 2025

(B) Activities to be started in 2025

Action: Lead

Guideline on the clinical requirements for sickle cell disease

Target date Publish guideline for public consultation Q4 2025

Action: Lead

Guideline on the clinical requirements for beta thalassemia

Target date Publish guideline for public consultation Q4 2025

2.2. Training and workshop activities

- Organise online webinars via the EUNTC platform on a regulatory topic concerned with plasma or other scientific aspects in the field of non-malignant haematology.

2.3. Communication and Stakeholder activities

2.3.1. European level

- Provide support (as relevant) on the increase of plasma donation and supply and liaise accordingly with the SPOC working Party, BWP, the EC SOHO team, EDQM and relevant stakeholders.
- Cooperation with relevant learned societies.
- Cooperation with the EC SOHO team, EDQM and ECDC on any plasma related matters.
- Participation of EMA to the joint meetings of the Competent Authorities on Blood and Blood Components, Tissues and Cells and Organs as needed.
- Participation of EMA to ECDC SoHONet meetings as observer.
- Cooperation with relevant patients' organisations.

2.3.2. International level

- Active contribution to the Blood cluster TCs with FDA (CBER) and Health Canada to discuss specific and identified issues from medicines under development, evaluation of products, global plasma issues as well as guidelines with the aim of achieving regulatory convergence in the clinical development of medicines under development, surveillance and regulatory actions (safety issues).
- Organise ad hoc TC with FDA and HC on Haematology products (not covered by Blood cluster).

2.4. Multidisciplinary collaboration

Given the broad spectrum of indications for immunoglobulins and their possible further expansion into other medical areas, also the importance of quality-related aspects for clinical evaluation and the nature of products in scope of this WP, interaction with other committees/WPs is needed (e.g. BWP, Methodology Working Party, the Paediatric Committee (PDCO), CAT, the Pharmacovigilance Committee (PRAC), the Orphan Committee (COMP), the Clinical domain WPs as relevant).

3. Operational goals

3.1. Pre-submission activities

- Contribute to requests for Scientific Advice (SA) and Protocol Assistance (PA) when requested by SAWP/CHMP.
- Strengthened contribution to paediatric investigation plans (PIP) upon request of PDCO.
- Respond to request for input arising from the CHMP/COMP/PDCO/PRAC/CAT.

3.2. Evaluation and supervision activities

- Discuss and review marketing authorisation applications and post-authorisation evaluation procedures to understand issues which should be addressed in new or revised guidelines.
- Address issues related to the evaluation of the safety and benefit/risk of plasma derivatives used as ancillary substances in medical devices.
- Collaborate with BWP and the EDQM on efficacy and safety issues linked to quality.
- Provide support, as requested, to relevant bodies responsible for inspections activities, the management of quality defects, sampling and testing and addressing issues of supply (i.e. plasma and blood donations).
- Contribute to the evaluation of risk management plans for products in non-malignant haematological indications, provide input into discussion of pharmacovigilance issues and contribute to referral discussions upon request from CHMP/PRAC.
- Respond to request for input arising from the CHMP/PDCO/COMP/PRAC/CAT as requested.

4. List of Abbreviations

BWP	Biologics Working Party
CAT	Committee for Advanced Therapies
CHMP	Committee for Medicinal Products for Human Use
COMP	Committee for Orphan Medicinal Products
EC	European Commission
ECDC	European Centre for Disease Prevention and Control
EDQM	European Directorate for the Quality of Medicines & HealthCare
EMA	European Medicines Agency
ESEC	European Specialised Expert Community

169	FDA	Food and Drug Administration
170	HC	Health Canada
171	JEIF	Joint EMA/Industry Task Force
172	HAEMWP	Haematology Working Party
173	PA	Protocol Assistance
174	PDCO	Paediatric Committee
175	PIP	paediatric investigation plans
176	PMF	Plasma Master File
177	PRAC	Pharmacovigilance Risk Assessment Committee
178	RSS	Regulatory Science Strategy
179	SA	Scientific Advice
180	SOHO	Substances of Human Origin
181	TC	Teleconference