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Human Medicines Division

Consolidated work plan for the Haematology Working Party (HAEMWP) 2024

Chairperson: Daniela Philadelphy

Vice chair: vacant

Work plan period: November 2023 - December 2024

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1. 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 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 other products with specific therapeutic indications, such as haemophilia e.g. plasma-derived and their recombinant alternatives (rFVIIa, rFVIII, rFIX, rFX, rFXIII), and novel non-replacement haemophilia therapies. In addition, any products developed for non-malignant haematological conditions fall in the remit of HAEMWP. For some of these products, close collaboration with other working parties and committees is needed, as these are also in the remit of e.g. the Committee for Advanced Therapies (CAT) in case of gene therapies.

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 long-standing contact with patients' organisations, through which the HAEMWP is aware that there is increasing anxiety about possible shortages of essential plasma products (e.g., immunoglobulins), will be continued.

In haemophilia the expertise in the HAEMWP can be used efficiently for novel therapies. The HAEMWP had already successfully liaised with the CAT and Scientific Advice Working Party (SAWP) on gene therapy products and contributed to the RP on gene therapies for haemophilia to be used by clinical assessors and has drafted a guideline on haemophilia non-replacement therapies.

2. Tactical goals: activities/projects to deliver the strategic goals

2.1. Guideline activities

Revision of existing EU Guidelines:

- Guideline and core SmPC on Immunoglobulins (SC/IM) (SCIg/IMIg) (CHMP/BPWP/410415/2011 Rev. 1)
- Guideline and core SmPC on fibrin sealant (EMA/CHMP/BPWP/741603/2015, replacing CPMP/BPWP/1089/00), EMA/CHMP/BPWP/598816/2010 rev. 1)
- Guideline on FIX and core SmPC (EMA/CHMP/BPWP/144552/2009 rev. 2 Corr. 1) and (EMA/CHMP/BPWP/1625/1999 rev. 3)

New EU Guidelines:

- Guideline on non-replacement therapies for haemophilia (EMA/CHMP/136018/2023)
- Guideline or RP on sickle cell disease
- Guideline or RP on thalassemias

2.2. Communication and Stakeholder activities

- Cooperation with Registry Holders to coordinate data collection and evaluation (this is part of the Regulatory Science Strategy (RSS) 2025).
- Work on regulatory research projects: the uniqueness of the HAEMWP is that it brings people and knowledge together from the EU Member States (MS) that otherwise is difficult to achieve in particular in this field where substantial national diversity exists.
- Participate in the Joint EMA/Industry Task Force (JEIF) meeting (in conjunction with BWP if relevant) on pandemic preparedness.
- Provide support (as relevant) on 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)).
- Organise meeting with plasma industry associations to further continue discussing regulatory and clinical scientific matters.
- EMA to organise and chair on a rota basis the Blood cluster TCs with FDA 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).
- Cooperation with learned societies.
- Cooperation with the EC SOHO team, EDQM and ECDC.
- Cooperation with relevant patients' organisations.
- Participate in conferences as speakers/chair persons to promote guidelines and share regulators' positions.
- Publish articles on product evaluation as well as in areas of interest, set up a publication plan agreed by HAEMWP.

2.3. 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).

3. Operational goals: medicinal product-specific activities

3.1. Pre-Authorisation activities

- Contribute to reviewing SA and PA when requested by SAWP/CHMP.
- Contribution to paediatric investigation plans (PIP) upon request of PDCO. Of note, this area could be further strengthened.
- Respond to consultations 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.
- Work with BWP and the EDQM on efficacy and safety issues linked to quality.
- Support, as requested, to inspections activities, quality defects, sampling and testing and address issues of supply (i.e. plasma and blood donations).
- Contribute to evaluation of risk management plans for products in non-malignant haematological indications, input into discussion of pharmacovigilance issues and contribute to referral discussions upon request from CHMP/PRAC.
- Respond to consultations arising from the CHMP/PDCO/COMP/PRAC/CAT as requested.

Priorities for 2024

4. Guidelines

4.1. EU Guidelines

Action: Lead

Guideline on non-replacement therapies for haemophilia

Target date	Finalise guideline following public consultation - Q4 2024 Consultation with PDCO, PRAC and COMP

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	Draft guideline to be released for public consultation Q2 2024

Action: Lead

Guideline on the Clinical Investigation of Human Recombinant Factor IX Products (EMA/CHMP/BPWP/144552/2009 rev. 2 Corr. 1) and Guideline on the Core SmPCs for Human plasma-derived and recombinant coagulation Factor IX products and (EMA/CHMP/BPWP/1625/1999 rev. 3)

Target date	Finalise guideline following public consultation - Q1 2024

Action: Lead

Fibrin sealant core SmPC and clinical investigation guideline (EMA/CHMP/BPWP/741603/2015, replacing CPMP/BPWP/1089/00), EMA/CHMP/BPWP/598816/2010 rev. 1

Target date Draft guideline to be released for public consultation Q1 2025

Action: Lead

Guideline or RP on sickle cell disease

Target date Draft guideline or RP to be released for public consultation Q4 2024

Action: Lead

Guideline or RP on beta thalassemia

Target date Draft guideline or RP to be released for public consultation Q4 2024

5. Training for the network and knowledge building

- 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.

6. Contribution to dialogue and engagement with stakeholders and external parties

6.1. Workshops

- Organise a multi-stakeholder workshop on hemoglobinopathies (sickle cell disease and thalassemias) in 2024.

6.2. Collaboration with Interested parties and other stakeholders

- Organise meeting with the plasma industry associations to further continue discussing regulatory and clinical scientific matters.
- Liaise with industry on recombinant/biotechnological products.

7. European collaborations

- 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.

8. International activities

- EMA to organise and chair on a rota basis 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).