European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

London, January 2009
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CHMP Blood Products Working Party (BPWP) Work programme 2009

I Meetings scheduled for 2009-2010

2009:

Dates

26 – 27 February 2009 (plenary)

4 – 5 June 2009 (drafting)

1 - 2 October 2009 (plenary)

26 -27 November 2009 (drafting)

2010:

Dates

25 - 26 February 2010 (plenary)

27 – 28 May 2010 (drafting)

30 Sep – 1 Oct 2010 (plenary)

25 – 26 November 2010 (drafting)

II. **Product related issues** (such as support to Marketing Authorisation Assessment, Post-marketing Data Evaluation, Scientific Advice, Protocol Assistance, Paediatric investigation plans including deferrals and waivers)**.**

In relation to the efficacy and safety of blood products, in agreement with the CHMP, the BPWP:

- Participates in assistance sought for Orphan Products.
- Reviews scientific advice sought by the industry, including advice related to paediatric use.
- Addresses issues related to the evaluation of medicinal products.
- Responds to consultations arising from the Paediatric Committee.

The following table provides the expected number per year of contribution (number of involvements in dossiers) for Scientific Advice, Protocol Assistance, Product Assessment and Post-Authorisation issues (including pharmacovigilance issues related to a product or a class of product).

In 2009, joint work with the PhVWP on Factor VIII inhibitors will continue.

	Expected contribution in Scientific Advice[1]	Expected contribution in Protocol Assistance	Expected contribution in Product Assessment	Expected contribution in Post-authorisation issues
2009	**11**	**9**	**5**	**9**

In agreement with the CHMP, the BPWP will address issues related to:

[1] Increase in scientific advice and protocol assistance expected related to the Paediatric Regulation

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- The evaluation of the safety and usefulness of blood derivatives used as ancillary substances in medical devices.
- The clinical evaluation of:
    - Novel therapies such as gene therapy for haemophilia and immunodeficiencies
    - Modified recombinant blood products (e.g. modifications to change pharmacokinetics)
    - Blood products manufactured using transgenic animals or plants
    - New routes of administration for blood products (e.g. inhalation)
    - Risk management plans for blood products.

III. CPMP/CHMP Guidance documents

1. Guidelines

Guidelines to be finalised after consultation period

1.1 Note for guidance on the Clinical investigation of recombinant Factor VIII and IX products (CPMP/BPWG/1561/99) and the Note for guidance on the Clinical investigation of human plasma derived Factor VIII and IX products (CPMP/BPWG/198/95 rev. 1)

Action: *Revision of the guidelines after experience with their use. Concept paper released for consultation in 2004. Workshop on Factor VIII inhibitors in 2006. Report of Workshop published in 2007. Revision of guidelines released for consultation in 2007 and expected to be finalised in early 2009.*

Guidelines to be reviewed after experience with their use:

1.2 Note for guidance on the Clinical investigation of Human normal immunoglobulin for intravenous administration (IVIg) (CPMP/BPWG/388/95 rev 1)

Action: *Revision of the guideline after experience with its use and in line with clinical developments. Discussion on the established indications. Concept paper released for consultation 2005. Workshop in 2006. Report of Workshop published in 2008. Revision of guideline expected to be finalised in 2009.*

1.3 Note for guidance on the Clinical investigation of Human normal immunoglobulin for subcutaneous and intramuscular use (CPMP/BPWG/283/00)

Action: *Review and possible revision of the guideline after experience with its use. Concept Paper 2010. Revision expected to be released for consultation in 2010.*

Guidelines to be prepared:

1.4 Guideline on the Clinical investigation of alpha$_1$-proteinase inhibitor (alpha$_1$ antitrypsin)

Action: *Concept Paper in 2009.*
Other involved WP: EWP

1.5 Guideline on non-clinical testing for blood products

Action: *Consideration of whether guideline is needed. On hold pending revision of ICH S6.*
Other involved WP: SWP

1.6 Guideline on the Clinical investigation of recombinant Factor VIIa (eptacog)

Action: *If guideline needed, Concept Paper in 2009.*

1.7 Guideline on the Clinical investigation of human C1 inhibitor

Action: *If guideline needed, Concept Paper in 2009.*

1.8 Guideline on the Clinical investigation of Human specific immunoglobulins

Action: *Concept Paper in 2009. Other involved WP: EWP.*

Guidelines of particular relevance to BPWP:

1.9 Comparability and similarity:

Guideline on Similar biological medicinal products (CHMP/437/04)

Guideline on Comparability of biotechnology-derived medicinal products after a change in the manufacturing process - non-clinical and clinical issues (CHMP/BMWP/101695/06)

Guideline on Similar biological medicinal products containing biotechnology-derived proteins as active substance: non-clinical and clinical issues (CHMP/42832/05)

Guideline on immunogenicity assessment of biotechnology - derived therapeutic proteins (CHMP/BMWP/14327/06)

Action: *Comments as needed in relation to the maintenance of the guidelines and the development of specific guidance for classes of products. Liaison with the BMWP and BWP as needed.*

1.10 European Commission Guideline on Summary of Product Characteristics

Action: *Comments as needed in relation to the maintenance of the guideline. Liaison as needed.*

1.11 Paediatrics Regulation and related guidance

Action: *Review of BPWP guidance in the light of the new legislation on Paediatrics.*

1.12 Activities of BWP in relationship to blood products

Action: *Liaison as needed with BWP on issues of common interest including viral risk assessment methodologies.*

2. Core Summaries of Product Characteristics (SPC) (revisions and new)

Core SPCs to be finalised after consultation period:

2.1 Core SPC for Human plasma derived and recombinant coagulation Factor VIII products (CPMP/BPWG/1619/99) and Core SPC for Human plasma derived and recombinant coagulation Factor IX products (CPMP/BPWG/1625/99)

Action: *Revision of the core SPCs after experience with their use. Concept paper released for consultation in 2004. Workshop on Factor VIII inhibitors in 2006. Report of Workshop published in 2007. Revised core SPCs released for consultation in 2007. Revision of core SPCs expected to be finalised in early 2009.*

2.2 Core SPC for Human normal immunoglobulin for intravenous administration (IVIg) (CPMP/BPWG/859/95 rev 2)

Action: *Revision of the core SPC after experience with its use and in line with clinical developments. Discussion on the established indications. Concept paper released for consultation 2005. Workshop in 2006. Report of Workshop published in 2008. Revision of core SPC expected to be finalised in 2009.*

Core SPCs to be reviewed after experience with their use:

2.3 Core SPC for Human normal immunoglobulin for subcutaneous and intramuscular use (CPMP/BPWG/282/00)

Action: *Review and possible revision of the core SPC after experience with its use. Concept Paper 2010. Revision expected to be released for consultation in 2010.*

2.4 Warning on transmissible agents for SPCs and patient leaflets

Action: *Comments as needed in relation to the maintenance of the guideline. Discussion on whether to make specific reference to vCJD. (Decision in 2008 that no warning needed where albumin is used as excipient).*

Other involved WP(s): BWP.

2.5 Core SPC for Human albumin solution (CPMP/PhVWP/BPWG/2231//99/Rev. 2)

Action: *To be kept under review in the light of new scientific information.*

Core SPC to be prepared:

2.6 Guideline on the Core SPC for alpha$_1$-proteinase inhibitor (alpha$_1$ antitrypsin)

Action: *Concept Paper in 2009.*
Other involved WP: EWP

2.7 Guideline on the Core SPC for human C1 inhibitor

Action: *If guideline needed, Concept Paper in 2009.*

IV. ICH Guidelines and activities

The working party shall contribute to applicable ICH guidelines under development that are identified after adoption of this work plan.

ICH S6 – Pre-clinical safety evaluation of biotechnology-derived pharmaceuticals

Action: *Comments as needed in relation to the revision of the guideline. Liaison with SWP as needed.*

V. EU Regulatory Activities

Medical devices incorporating blood derivatives as ancillary substances, activities as needed.

VI. Activities with external parties

Consultations as appropriate with Industry (including IPFA and PPTA), Patients' organisations (including EHC and EPPIC) and organisations of health professionals (including ISTH and ESID).

International cooperation as appropriate (including WHO, FDA, Japan and Canada).

In addition to the specific areas already listed in this document, BPWP will continue to maintain an interest in patient registries and their potential as a source of valuable information on the clinical use of blood products.

VII. Organisational matters

Organisational matters as appropriate to support the work of the BPWP.

APPENDIX I

Status of old core SPC documents[2]:

Human plasma coagulation Factor VIII Concentrate *(update adopted)*

Human plasma coagulation Factor IX Concentrate *(update adopted)*

Human plasma coagulation Factor VII Concentrate *(update adopted)*

Human plasma coagulation Factor XIII Concentrate *(not to be updated due to small number of products, no longer valid)*

Human plasma prothrombin complex concentrate *(update adopted)*

Human plasma Antithrombin III Concentrate *(update adopted)*

Human plasma anti-inhibitor coagulant complex concentrate *(not to be updated due to small number of products, no longer valid)*

Human plasma Fibrinogen Concentrate *(update adopted)*

Human albumin *(update adopted)*

Human normal immunoglobulin i.v. *(update adopted)*

Human varicella immunoglobulin i.v. *(update adopted)*

Human cytomegalovirus immunoglobulin i.v. *(not to be updated, no longer valid)*

Human normal immunoglobulin i.m. *(update adopted)*

Human rabies immunoglobulin i.m. *(update adopted)*

Human tetanus immunoglobulin i.m. *(update adopted)*

Human anti-D immunoglobulin i.m. *(update adopted)*

Human hepatitis B immunoglobulin i.m. (*update adopted)*

Human tick-borne encephalitis immunoglobulin i.m. *(update adopted)*

Human measles immunoglobulin i.m. *(not to be updated, no longer valid)*

Human rubella immunoglobulin i.m. *(not to be updated, no longer valid)*

[2] Published by European Commission in 1992

APPENDIX II

Current BPWP Guidance documents

Adopted Guidelines

CPMP/BPWG/198/95 Rev. 1 Note for Guidance on the Clinical Investigation of Human Plasma Derived Factor VIII and IX Products (Adopted October 2000, into effect April 2001))*

CPMP/BPWG/1561/99 Note for Guidance on the Clinical Investigation of Recombinant Factor VIII and IX Products (Adopted October 2000, into effect April 2001)*

CPMP/BPWG/1619/99 Core SPC for Human Plasma Derived and Recombinant Coagulation Factor VIII Products (Adopted June 2000, into effect December 2000)*

CPMP/BPWG/1625/99 Core SPC for Human Plasma Derived and Recombinant Coagulation Factor IX Products (Adopted June 2000, into effect December 2000)*

CPMP/BPWG/575/99 Rev. 1 Guideline on the Clinical investigation of human anti-D immunoglobulin for intravenous and/or intramuscular use (Adopted September 2007, into effect 1 April 2008)

CPMP/BPWG/574/99 rev. 1 Guideline on the Core SPC for Human anti-D immunoglobulin for intramuscular use (Adopted September 2007, into effect 1 April 2008)

CHMP/BPWP/319619/2005 Guideline on the Core SPC for Human anti-D immunoglobulin for intravenous use - Revision 1 (Adopted September 2007, into effect 1 April 2008)

CPMP/BPWG/2220/99 Note for Guidance on the Clinical Investigation of Plasma derived Antithrombin Products (Adopted January 2002, into effect July 2002)

CPMP/BPWG/3226/99 Core SPC for Human Plasma derived Antithrombin (Adopted January 2002, into effect July 2002)

CPMP/BPWG/283/00 Note for Guidance on the Clinical Investigation of Human Normal Immunoglobulin for Subcutaneous and Intramuscular use (Adopted July 2002, into effect January 2003)

CPMP/BPWG/282/00 Core SPC for Human Normal Immunoglobulin for Subcutaneous and Intramuscular use (Adopted July 2002, into effect January 2003)

CPMP/BPWG/BWP/561/03 Note for Guidance on the Warning on Transmissible Agents in SPCs and Package Leaflets for Plasma-derived Medicinal Products (Adopted October 2003, into effect April 2004)*

CPMP/BPWG/1089/00 Note for Guidance on the Clinical Investigation of Plasma derived Fibrin Sealant Products (Adopted July 2004, into effect January 2005)

CPMP/BPWG/153/00 Core SPC for Plasma derived Fibrin Sealant Products (Adopted July 2004, into effect January 2005)

CPMP/BPWG/388/95 Rev. 1 Note for Guidance on the Clinical Investigation of Human Normal Immunoglobulin for Intravenous Administration (IVIg) (Adopted June 2000, into effect December 2000)*

CPMP/859/95 Rev. 2 Core SPC for Human Normal Immunoglobulin (IVIg) for Intravenous administration (Adopted July 2004, into effect November 2004)*

CPMP/BPWG/2048/01 Core SPC for Human Plasma Coagulation Factor VII Products (Adopted July 2004, into effect January 2005)

CPMP/BPWG/3735/02 Core SPC for Human Prothrombin Complex Products (Adopted October 2004, into effect April 2005)

* Under revision

CPMP/BPWG/3728/02 Core SPC for Human Rabies Immunoglobulin for Intramuscular Use (Adopted July 2005, into effect February 2006)

CPMP/BPWG/3730/02 Core SPC for Human Tetanus Immunoglobulin for Intramuscular Use (Adopted July 2005, into effect February 2006)

CPMP/BPWG/3732/02 Core SPC for Human Tick-Borne Encephalitis Immunoglobulin for Intramuscular Use (Adopted July 2005, into effect 1 February 2006)

CPMP/BPWG/3726/02 Core SPC for Human Varicella Immunoglobulin for Intramuscular Use (Adopted July 2005, into effect 1 February 2006)

CPMP/PhVWP/BPWG/2231/99 Rev. 2 Core SPC for Human Albumin (Adopted November 2005, into effect 1 June 2006)

CPMP/BPWG/220/02 Note for Guidance on the Clinical Investigation of Human Plasma Derived von Willebrand Factor Products (Adopted November 2005, into effect 1 June 2006)

CPMP/BPWG/278/02 Core SPC for Human Plasma Derived von Willebrand Factor (Adopted November 2005, into effect 1 June 2006)

CPMP/BPWG/4222/02 Core SPC for Human Plasma Derived Hepatitis-B Immunoglobulin for Intramuscular Use (Adopted April 2006, into effect 1 November 2006)

CPMP/BPWG/4027/02 Core SPC for Human Plasma Derived Hepatitis-B Immunoglobulin for Intravenous Use (Adopted April 2006, into effect 1 November 2006)

CHMP/BPWP/122007/05 Core SPC for Human Fibrinogen Products (Adopted January 2009, into effect August 2009)

Draft Guidelines

(Includes revision to previously adopted guidelines)

CPMP/BPWG/198/95 rev.2 Guideline on the Clinical Investigation of Human Plasma-Derived Factor VIII and IX Products

CPMP/BPWG/1561/99 rev.1 Guideline on the Clinical Investigation of Recombinant Factor VIII and IX Products

CPMP/BPWG/1619/99 rev.1 Guideline on the Core SPC for Human Plasma-Derived and Recombinant Coagulation Factor VIII Products

CPMP/BPWG/1625/99 rev.1 Guideline on the Core SPC for Human Plasma-Derived and Recombinant Coagulation Factor IX Products