Platform of Plasma Protein Users

25 October 2013

ABO RBDM Framework Project, Stakeholders Consultation Committee
Att. Ms. Lorna Lemay
National Director, Stakeholder Relations & Translation
Canadian Blood Services

Dear Ms. Lemay,

PLUS Open Letter to ABO Risk-Based Decision Making

PLUS, the Platform of Plasma Protein Users, represent organisations of patients with treatable rare diseases linked by common therapies based on products manufactured from human plasma.

Every year since 2010, PLUS convenes Consensus Stakeholders meetings. Key stakeholder organisations active in the field of blood and plasma derived medicinal products participate with a view to discuss developments affecting our patient communities and to identify consensus principles.

This year, the PLUS Stakeholders Consensus Meeting was held in Estoril (Portugal) on 5-6 September.

Two topics were explored and discussed at the meeting:

- The development of a risk management strategy and framework for blood and plasma derived medicinal products.
- Treatment in 2020: Patients Views

Experts were invited to provide presentations on the topics which were then discussed.

The participants in attendance represented the following organisations: Alpha-1 Foundation, CEREDIH (French PID National Reference Centre), European Blood Alliance, European Haemophilia Consortirum, European Medicines Agency, International Federation of Blood Donor Organisations, International Patient Organisation for Primary Immunodeficiencies, Irish Blood Transfusion Service, International Plasma Fractionation Association, PLUS Steering Committee, Plasma Protein Therapeutics Association, World Federation of Haemophilia.

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On the basis of these discussions, several consensus principles were identified and agreed by the participants at the meeting (It should be noted however that, unlike previous consensus meetings organised by PLUS this does not constitute a formal consensus statement which would require endorsement by the organisations represented by those in attendance).

1. **The role of patients' organisations in the development of a risk management strategy and framework for blood and plasma derived medicinal products.**

   Principles

- The process undertaken to evaluate the risk-benefit equation is fundamentally important to having an adequate, safe and affordable supply of blood and plasma derived medicinal products (PDMPs).

- We recognize that zero risk is almost always unattainable. Development of an integrated risk management strategy could be a significant first step to establish a framework for managing the interrelationship of risk tolerance and measurement, supply of blood and plasma derived medicinal products and economic considerations.

- The precautionary principle is a fundamental component of risk-based decision making. However, it may not be appropriate in all situations. It should only be applied where there is uncertainty. When it is applied, it is essential that the final framework takes into account each of the components of the precautionary principle and promotes a process for their appropriate application.

- The participants concurred with the importance of developing an integrated, internationally applicable framework, entrenched in donor safety and optimal patient outcomes, to guide major policy and operational change.

- Patients whose continued health is dependent upon blood components or PDMPs have a right, through their representative organisations, to be consulted on any issue which may have an impact on the safety, efficacy or supply of the treatment they receive.

- To this end, stakeholder engagement to include patient organisations input within the framework is essential, both in its development and implementation.

- The PLUS consensus process would be pleased to serve as a resource for the framework development process and provide coordinated input from the

constituent organisations of PLUS and those joining in the annual consensus process.

2. Treatment in 2020: Patient and Physician Views

In the discussions that followed the presentations on the topic of "Treatment in 2020", several elements were identified.

Although the purpose of this session was not directly related to the topic of risk-based decision making, the following key elements that emanated from the discussions are nevertheless also relevant and should be embedded in a risk management framework and strategy.

Elements common to all groups:

- Patient and donor safety
- Plasma supply management
- Access to therapies (includes issues of competing markets, costs and prices)
- Detection and early diagnosis
- Education of patients to improve health status and compliance/adherence to treatment. Comprehensive health management
- Appreciation of the interdependence among the needs of individuals requiring treatment with PDMPs
- The ethical and practicable design of clinical trials for new treatments
- Political visibility and involvement (advocacy)
- Comprehensive patient-centred outcomes analysis
- Recognition of the global context
- Genetic counselling (*this is relevant for a majority of rare disorders represented by PLUS*)

Elements of particular importance to the haemophilia community

- Treatment evolutions & development
- Costs & prices – Reimbursement policies
- Ageing
- Standardised treatment guidelines (Europe)
- Collection of outcome Data

Elements of particular importance to the PID community

- Prioritization and access; increase in availability of reference centres
- Therapeutic evolution and advance

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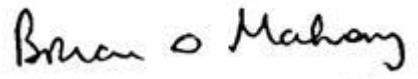
- Holistic approaches
- Stakeholder collaboration; patient awareness and advocacy; political visibility and presence
- Newborn screening
- Registries (data); networks; education; critical mass
- Ageing issues
- Global approach

Elements of particular importance to the Alpha 1 community

- Access to therapies; (issues of competing markets)
- Early diagnosis and detection
- Comprehensive health management beyond the HTC model – coaching, peer health co-ordinating, confluence
- Use of technology to manage care-Apps, iPAds and pedometers
- The ethical design of clinical trials in alpha 1 antitrypsin deficiency.

We hope that the above-mentioned information will be helpful and would encourage you to integrate the views of the PLUS consensus platform stakeholders into ABO's RBDM framework project.

Yours sincerely,



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