

Ref.: DGSANCO14003
10 April 2014

The PPTA vision on the plasma protein therapies sector for the next decade in Europe

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EXECUTIVE SUMMARY

The present paper has been developed by the Plasma Protein Therapeutics Association (PPTA) upon request of the European Commission DG Sanco - Substances of Human Origin Unit. This is to inform the EU Commission about PPTA's vision on future perspective of the plasma industry sector. The present paper contains several industry considerations in view of a potential 2002/98/EC Directive reform.

- Most of the patients treated with plasma derived therapies are affected by rare diseases. These are caused by genetic defects which manifest themselves in early childhood and are often life-threatening. Therefore, they require patients to receive regular infusions or injections of plasma protein therapies. Due to the constant improvement in diagnosis and the extension of life-expectancy **more patients need and will need more therapies**. The clinical demand for these medicines has always increased over the past years and, as further developed in this paper, this will continue. Therefore, **more plasma for fractionation will be needed** to manufacture the essential therapies that patients need.
- To collect plasma for fractionation both plasma “recovered” from whole blood and source plasma are required. However, the supply trend of recovered plasma is flat due to diverse driving reasons behind its usage. Nowadays, the vast majority of the collected plasma used for fractionation comes from source plasma. Therefore, it is important for the **European Union to recognize the intrinsic difference between whole blood/blood components for transfusion and plasma for fractionation and to implement targeted policies to encourage plasma collection and raise awareness on the importance of donating plasma for fractionation**.
- The EU **should further differentiate within its legislation between whole blood/labile blood components intended for transfusion and the collection of plasma intended for fractionation**. This will reduce ambiguity in the EU law implementation and consequently will prevent the introduction of national measures which, without a real public health justification, restrict the access of EU internal market benefits to patients. More efforts to increase competitiveness in Europe should be undertaken in order to ensure that patients have a stable access to therapies.
- Since the ultimate goal of guaranteeing a safe and stable supply of plasma and plasma derived medicinal products is to ensure patients adequate access to care, PPTA strongly **recommends taking into consideration patients' perspective as any reform will have an impact on patient access to care**.

RECOMMENDATION FOR A POTENTIAL REVISION OF THE EU BLOOD DIRECTIVE 2002/98

Should the European Commission consider reforming the current EU Blood Directive 2002/98, PPTA recommends that:

- The new EU legislation reflects the difference between whole blood/labile blood components for transfusion and plasma for fractionation
- The ultimate objective when regulating the plasma collection system should be that the use of plasma protein therapies should be driven by the patient's clinical need
- Self-sufficiency for whole blood and labile blood components is a realistic goal
- Self-sufficiency for plasma protein therapies is not a realistic goal
- Compensation of source plasma donors for their time and inconvenience should be clearly recognized as compatible with Voluntary Unpaid Donation
- The scope of the EU Blood Directive should be clarified in line with the interpretation of the European Court of Justice in the case of Octapharma France versus ANSM (C-512/12) to remove national barriers that restrict patient access to plasma protein therapies

INTRODUCTION

The PPTA is the international trade association and standards setting organization representing the world's major collectors of plasma for fractionation and manufacturers of plasma derived therapies and recombinant analogues, collectively known as plasma protein therapies, which are used in the treatment of rare diseases.

These diseases are often genetic, chronic, life threatening and require patients to receive regular infusions or injections of plasma protein therapies. The **therapies include:**

- **Immunoglobulins** to treat a number of diseases in individuals with immune-deficiencies and autoimmune diseases
- **Clotting-factor** therapies for individuals with hemophilia A and B and other bleeding disorders
- **Albumin**, which is used in acute settings to treat individuals with shock, trauma, burns and other conditions
- Therapies for individuals who have **alpha-1 anti-trypsin deficiency**, which typically manifests as adult onset emphysema and limits substantially life expectancy
- Therapies for individuals with protein C deficiency, a rare life-threatening genetic disease that predisposes to thrombotic disease

Plasma protein therapies are unique, biologic medicines that are either infused or injected to treat a variety of rare, life-threatening, chronic and genetic diseases.

For over 60 years, the plasma protein industry has played a pioneering role in treating rare genetic diseases. The plasma protein industry has invested in the development of medicines for treating rare diseases before the introduction of policies stimulating the development of orphan drugs. Thanks to the enormous investments made by this industry, patients have seen increased life expectancy and improvements in their quality of life.

In recent years, considerable attention has been paid to stimulate the research, development and marketing of medicinal products for rare diseases. However, despite these positive developments, difficulties in treating rare diseases still persist.

The challenges to treat rare diseases are many. Patient numbers are low and patients are usually geographically widely dispersed. Patients are often not timely diagnosed and medical expertise is limited or scattered.

Often patients with plasma protein deficiency across Europe see access to therapies denied or delayed. The diverse clinical trials requirements and processes for assessing new medicines (e.g. HTA) for the treatment of rare diseases in different Member States make rapid and equitable access across the EU difficult to achieve.

Legislative proposals as well as additional voluntary actions at EU and Member State level should be focused on improving access to plasma protein therapies in a coordinated manner.

Continued dialogue and engagement from all stakeholders, including patients and industry, is an important prerequisite to maintain and further develop a solid legislative framework and strategic plans for implementation. PPTA and its members are committed to playing their part.

The clinical need for these therapies is growing and requires more plasma and increased plasma fractionation capacity in the future (1). PPTA is happy to provide the European Commission with its vision on how to ensure best the life-saving or life-enhancing plasma protein therapies that our members manufacture.

THE STARTING MATERIAL

The PPTA members are committed to providing safe, effective, life-saving plasma protein therapies to patients around the world. To manufacture these therapies human plasma is used as starting material. This plasma can be obtained in two different ways: recovered plasma from whole blood donations and source plasma from plasmapheresis donations. Both sources are needed to manufacture therapies in sufficient quantities to help the many patients in the world whose life depend on these therapies.

Whole Blood

Whole blood for transfusion and preparation of fresh components is mainly collected by national organizations and in some instances (e.g. Germany) also by private organizations.

The donations come primarily from Voluntary Unpaid Donors (VUD), although interpretations of this concept vary. In Germany, for example, compensation is allowed by law up to Euro 25 per donation (Aufwandsentschaedigung) and this is considered compatible with VUD.

The average donation frequency per donor is 1 - 2 times per year. The blood donation takes up to half an hour. Donations are given in different settings, like blood banks, hospitals, mobile units and collection centers.

It is well understood that the short shelf lives and particular storage conditions of the labile components derived from whole blood impose restrictions on the level to which these can be transported safely across long distances. This makes sufficiency of these products from national sources an important consideration for health care systems. Such a level of sufficiency has been attained within most of countries of the European Union to a certain extent. This is ensured in some countries through the decrease in use, particularly of red cells, experienced in the era of Patient Blood Management (PBM).

The European Blood Directive 2002/98 provides guidance in the area of Whole Blood and in transfusion practices. PPTA is not involved in transfusion medicine and refrains from detailed comments on the technicalities of whole blood in transfusion.

Recovered Plasma

If whole blood collections and its components are not 100% used for transfusion in hospitals, it can still be used for fractionation under the term recovered plasma. This recovered plasma must comply with the European Pharmacopeia Monograph n 853, “Human Plasma for Fractionation,” and with all applicable EU regulations regarding the manufacture of plasma protein therapies. Recovered plasma generates income for the blood banking/collection sector (2).

Safety and efficacy concerns of allogeneic blood transfusions and their impact on patient outcomes have resulted in the growth worldwide in PBM efforts (3). The PBM focuses on multidisciplinary and multimodal preventive measures to reduce or obviate the need for transfusions and ultimately to improve the clinical outcomes of patients (3). Because of the implementation of PBM, a decrease in the need for whole blood and blood components for transfusion will likely lead to a decrease in availability of recovered plasma and may result in lower revenue for the blood banking sector.

Linking the supply of plasma protein therapies only to the availability of recovered plasma would risk over-collection of blood in order to keep up with the growing clinical needs for plasma protein therapies, resulting in wastage of red cells and an unethical hazard of donor health through iron deficiency (4). Alternatively, gearing the supply of plasma protein therapies (5) to the limited plasma supply recoverable from a blood collection system optimized to red cell needs will impede the growing clinical needs for plasma protein therapies (6). Therefore, it is inevitable that a system ensuring the clinical needs of all patients – transfusion recipients in hospitals and chronic recipients of plasma protein therapies – has to provide components through blood collection and plasma for fractionation through dedicated plasmapheresis.

In Europe and in North America, it can be seen that the volume of recovered plasma used for fractionation is not growing but is flat. Growth in the availability of plasma for fractionation comes from source plasma.

Figure 1: MRB - Type of Plasma processed in Europe from 1996 to 2012 commercial companies & non-profit organizations (Thousand Liters)

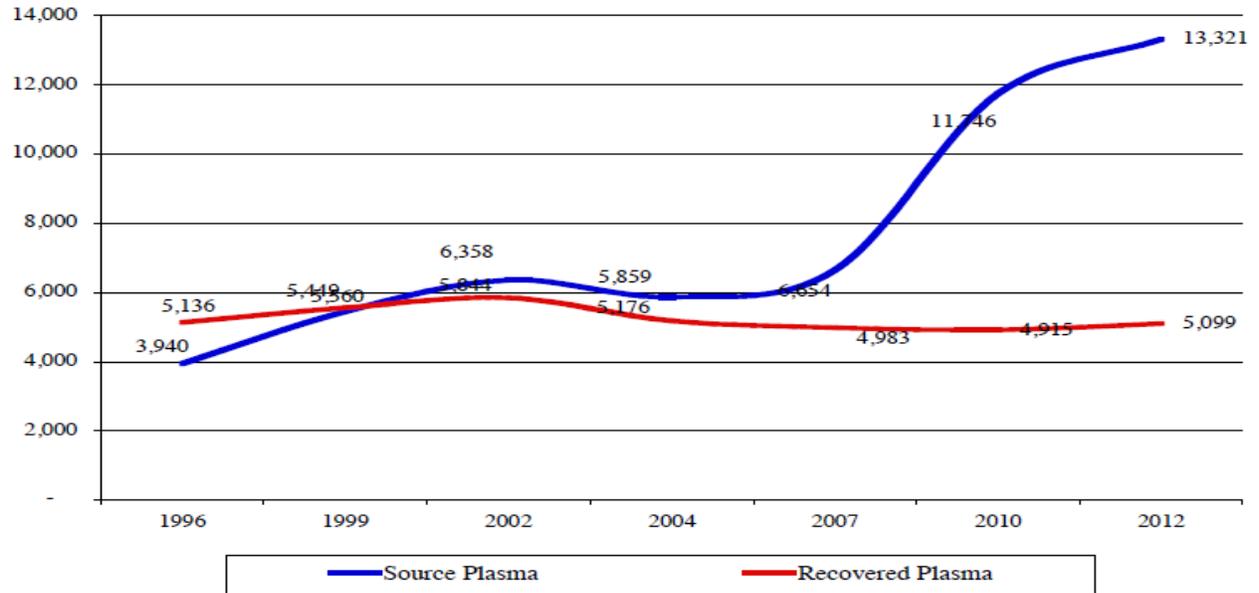
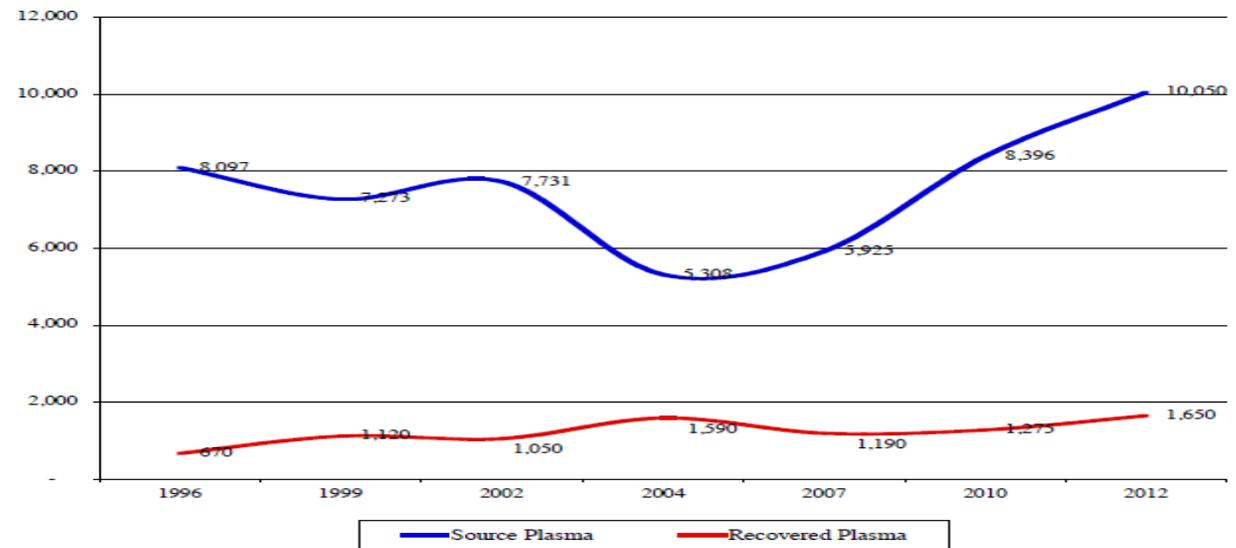


Figure 2: MRB - Type of Plasma processed in North America from 1996 to 2012 commercial companies & non-profit organizations (Thousand Liters)



Source Plasma

A source plasma donation is typically done via apheresis (plasmapheresis). A plasmapheresis donation takes a longer time than a whole blood donation but can be performed more frequently.

The intended purpose of plasma obtained by apheresis is mainly fractionation, an industrial process of separating therapeutic proteins for the manufacture of stable pharmaceutical products with a defined shelf life. Apheresis per se is never mentioned in the pharmaceutical legislation and European Pharmacopoeia Monograph n. 853; they talk about “processing”; collection is still within the scope of the Blood Directive. The pharmaceutical legislation and the European Pharmacopoeia cover plasma, whatever its method of collection, when it is used as pharmaceutical starting material.

Processing of plasma for fractionation and the manufacture of plasma derived therapies is a strictly regulated process covered by the Eur. Ph. Monograph n. 853, Directive 2001/83/EC as amended and Annex 14 of the GMPs. Experience in the source plasma collection sector has shown that donors who live in the community, in which they donate, are committed to the plasma collection program: they donate frequently and are the high quality donors. PPTA has developed and implemented the Qualified Donor Standard as a voluntary industry initiative to ensure that only plasma from regular and committed donors is entering the manufacturing process (7).

Several Member States have introduced regulations which allow financial compensation for donors for their time and efforts/inconvenience (8) (9) (10). Analysis of blood and plasma collection trends shows that countries compensating plasma donors are the highest providers of plasma for fractionation (11) (12).

Policies to encourage citizens to donate plasma vary among Member States and this is due to a different interpretation of the VUD principle. While some Member States reject any form of financial compensation, some others, such as Germany, Austria, Czech Republic and Hungary consider that financial compensation for time and inconvenience to donate plasma is compatible with the VUD principle. PPTA supports an interpretation of the VUD principle that includes compensation of donors for their time and inconvenience. This is also consistent with the EU Directive 2004/23/EC on tissues and cells that recognizes the need to compensate donors for their expenses and inconvenience.

Considering the flat supply of recovered plasma, source plasma is the essential source for the further growth to meet clinical needs of plasma protein therapies, which are used for treating chronic and often life-threatening rare genetic diseases. Patients affected by these severe rare diseases, such as immunodeficiency, hemophilia, protein C

deficiency, or alpha-1 antitrypsin deficiency, rely heavily on regular access to these medicines. The time between a source plasma donation and a finished plasma protein therapy to patients takes seven-to-nine months. In order to guarantee a continuous access to these life-savings therapies it is important that an uninterrupted plasma supply is ensured.

Therefore, any policy measure which could potentially exclude some kind of donations will have a dramatic impact on the supply of plasma protein therapies and, on the life and well-being of patients depending on these therapies.

It is important for the European Union to recognize the intrinsic difference between blood and plasma donation and to implement targeted policies to encourage plasma donations and raise awareness on the importance of donating plasma for fractionation¹.

Unfortunately, this difference is not recognized in the outcomes of a recent meeting among selected WHO and other experts in Rome (13). At the end of this meeting, a WHO Declaration on “Achieving self-sufficiency in safe blood and blood products, based on voluntary and non-remunerated donation” has been released. In this paper, WHO experts call nations “*to phase out in a programmed manner, the use of blood components for transfusion, intermediates and PDMP (Plasma Derived Medicinal Products) obtained from paid or compensated donors...*”.

This statement contrasts with the European Medicines Agency’s Committee for Proprietary Medicinal Products (CPMP - now CHMP). The CPMP position statement (14) states that “both non-remunerated and remunerated donors contribute to the supply of safe plasma-derived medicinal products”. Consequently, **PPTA is concerned that the WHO statement may harm patients**, as it overlooks accepted scientific and medical evidence for effective treatment of these serious, chronic diseases, which require more products worldwide than are currently supplied (15) (16). Following the WHO’s statements will restrict collection and manufacture, causing product shortage, loss of treatment and adverse patient outcomes. This is also in contradiction with their own data (see Figure 3) where the vast majority of plasma for fractionation is compensated.

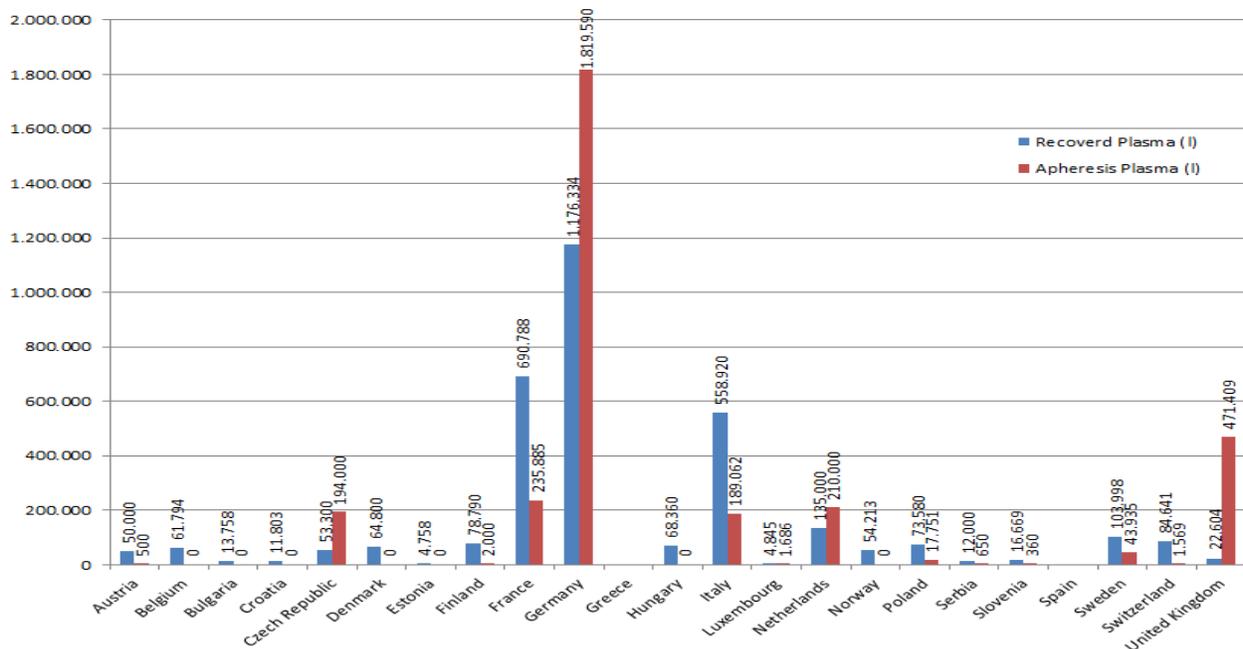
¹ Some Member States, such as Germany, Austria, Czech Republic and Hungary, provide compensation for plasma donations. The referenced countries believe that their approach to compensate for time and inconvenience is entirely compatible with the principle of VUD. However, the majority of Member States provide some type of incentive regardless of whether the donation is for blood or plasma. In fact, in Europe there are practically no differences between regulatory treatment of incentives offered for whole blood and plasma donation (see 2nd Report on Voluntary and Unpaid Donations of Blood and Blood Components, Brussels 23.3.2011, COM(2011)138 final).

Plasma for Fractionation

Plasma for fractionation can be collected either from whole blood or by plasmapheresis. Nonetheless, the vast majority of the collected plasma used for fractionation comes from source plasma (1).

The WHO data show the essential contribution of source plasma in order to satisfy the need for plasma for fractionation.

Figure 3: WHO data on plasma volume, recovered & apheresis (WHO Global Database on Blood safety 2011 data) sent for fractionation*



* PPTA comment: WHO data regarding Austria, Czech Republic and UK are not correctly reported. In 2011 Austria collected about 470.000 liters of source plasma (PPTA data) and Czech Republic collected 101.069 liters of recovered plasma and 459.296 liters of source plasma (Data from Institute of Health Information and Statistics of the Czech Republic). UK does not collect any Source Plasma within the UK territory because of vCJD policy.

The contribution of source plasma in order to satisfy the clinical need for plasma for fractionation is also noticeable in the EDQM data (34).

PPTA emphasizes that restricting plasma collection, either on a Member State or European level, is not appropriate in the case of plasma and stable medicinal products derived from plasma. Self-sufficiency is appropriate for labile blood components but not achievable for plasma and stable medicinal products derived from plasma.

PPTA also highlights that very strict regulatory requirements exist to guarantee the quality and safety of donations and of donors, irrespective of their origin. Limiting the geographic scope of the supply is an artificial, unneeded and

potentially damaging practice which could lead to the ultimate detriment of the patients.

Any legislative or regulatory document that puts whole blood/labile blood components for transfusion and plasma protein therapies into the same framework will provide additional room for confusion. PPTA recommends that a different approach shall be taken.

In summary, the desire for self-sufficiency in the arena of labile blood components is distinguishable from the picture of appropriate and effective access to stable medicinal products, such as plasma protein therapies. These two arenas are markedly different and necessitate different policy treatment.

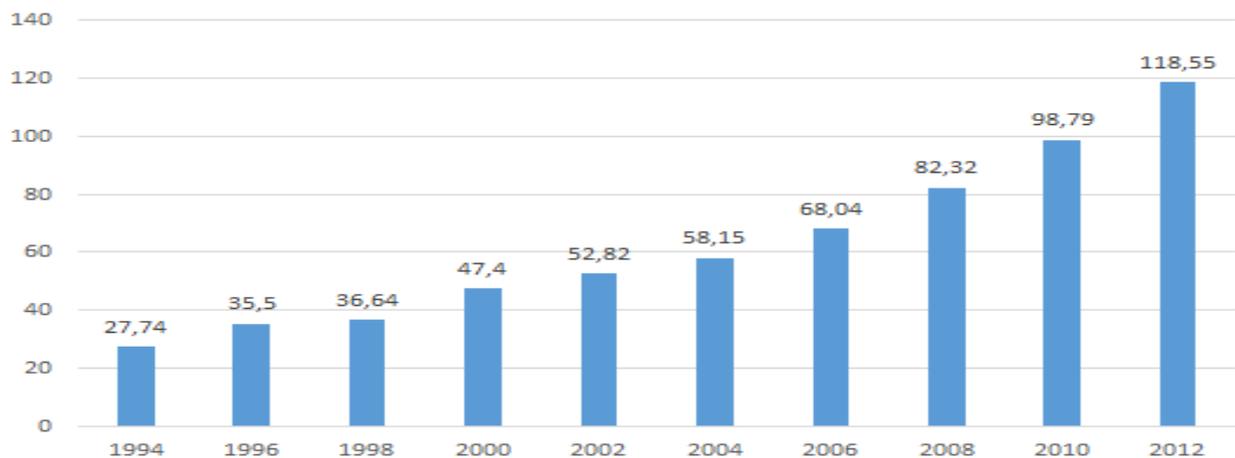
PLASMA PROTEIN THERAPIES

Plasma protein therapies are either plasma derived therapies or their recombinant analogues. PPTA supports the principle of freedom of choice for any therapy and believes this is a decision that needs to be made in concert between a physician and the patient. For the purpose of this document we focus on the plasma derived medicinal products.

Immunoglobulins

The need for immunoglobulins (IG) is the driver for the volume of plasma needed for fractionation.

Figure 4: MRB - Worldwide demand for polyvalent intravenous immunoglobulin (IVIg) 1994 – 2012 (Metric Tons)



Market Research Bureau estimates that the clinical need for immunoglobulins will increase (17).

Immunoglobulins are used to treat many rare disorders e.g. Primary Immune Deficiency (PID). For most people who are affected by PID, immunoglobulin is the only available care and unfortunately, the availability of therapies for immunodeficiency varies enormously (IPOPI (18)).

The paper “Modeling primary immunodeficiency disease epidemiology and its treatment to estimate latent therapeutic demand for immunoglobulin” by Stonebraker J. et al (16) shows that the potential clinical need for treating CVID (Common Variable Immune Deficiency) and XLA (X-linked agammaglobulinemia) exceeds the currently observed usage of IG in these disorders.

PPTA distribution data on plasma protein therapies in Europe highlights the fact that significant disparities in IG consumption persist at the national level (19). There is no evidence that these disparities in IG usage reflect actual underlying national disparities in disease prevalence, suggesting that many Europeans suffering from Primary Immune Deficiency or other IG-treatable conditions are currently receiving less IG therapy than is assessed in the Stonebraker J. paper (16).

There has been also tremendous progression in genetic research for characterizing and diagnosing immunodeficiency (e.g. team of A. Fischer in France). Better diagnosis leads generally to higher clinical needs.

Factor VIII

The global use of plasma derived FVIII (pdFVIII) has increased over the last decade (see pp. 22 in reference 24) although recombinant FVIII products have been made available. There are also currently a number of new recombinant FVIII products in development (20). However, most of these products are not yet registered.

A paper titled “Modelling hemophilia epidemiology and treatment modalities to estimate the unconstrained factor VIII demand” by Stonebraker J. states the following: “The probability-weighted average for the unconstrained FVIII demand model was 6.9 units per capita” (15). However, the average consumption in Europe is lower. They highlight as well the differences in FVIII consumption between countries and the potential for more state of the art treatment regimens such as prophylaxis. Since this publication, many countries have exceeded this projected demand as prophylactic and tolerisation protocols have improved. Restricting the access to any potential source of FVIII, whether from recombinant or plasmatic, will impede the continuing progress in haemophilia care. It should be pointed out that the vast majority of the world’s haemophilia population is untreated. Restricting a particular country’s plasma collection efforts to that which is required by its population contradicts the principle of human solidarity.

Albumin

Current global albumin production is estimated to be about 550 tons per year and worldwide clinical need is predicted to grow at 15% per year (see pp. 159 in reference 24) Albumin is widely recognized as a versatile therapeutic agent for a variety of diseases, including sepsis and cirrhosis, where its unique biological properties enhance its effectiveness relative to other therapies. Continuing clinical evidence indicates that the requirements for albumin in these disorders will increase.

New Plasma Proteins and investing in innovation

Quality and innovation have been the drivers of the development and success of the plasma protein industry to improve the quality of life of patients and to ensure donor safety.

In the recent years several innovations have been implemented in the manufacturing process (e.g. improvement of yield), product presentations (e.g. subcutaneous IG; IVIG 10% concentration; IVIG liquid presentation) additional virus removal technologies (e.g. nanofiltration) as well as the development of new plasma proteins (e.g. protein C and purified factor V).

Additionally, there is a continuous investment by the plasma protein industry in research and development of new plasma proteins therapies (e.g.: Ceruloplasmin, IgA, Plasmin, Protein S...) (21) that will lead to innovative treatments for the benefits of patients.

FACTS AND FIGURES

In 2012, 36.7 million liters of plasma were fractionated worldwide or 9.4% more than in 2010. This increase was predominantly attributed to source plasma (+40.9% 2010 vs 2007 - +8.9% 2012 vs 2010), as recovered plasma gained +3.8% in 2010 vs 2007 and +10.9% in 2012 vs 2010. The global volume comprised 27.8 and 8.9 million liters of source and recovered plasma respectively (1).

In 2012, the commercial sector processed 31.5 million liters of plasma, including 25.1 million liters of source plasma and 6.3 million liters of recovered plasma. For its part, the non-profit sector fractionated 5.2 million liters of plasma, including 2.6 million liters of source plasma, and 2.6 million liters of recovered (1).

The regional distribution of the throughput was the following (1):

- 18.4 million (50%) in Europe
- 11.7 million (32%) in North America
- 5.0 million (14%) in Asia

- 1.6 million (4%) in the rest of the world

Regarding the collection of plasma by the PPTA members, there is about 9 times more collection of plasma in the US compared to EU. The collection of plasma by our members has more than doubled between 2005 and 2012 in both US and EU (22).

Figure 5: PPTA data on source plasma collection in Europe (Gross Liters) by PPTA members*

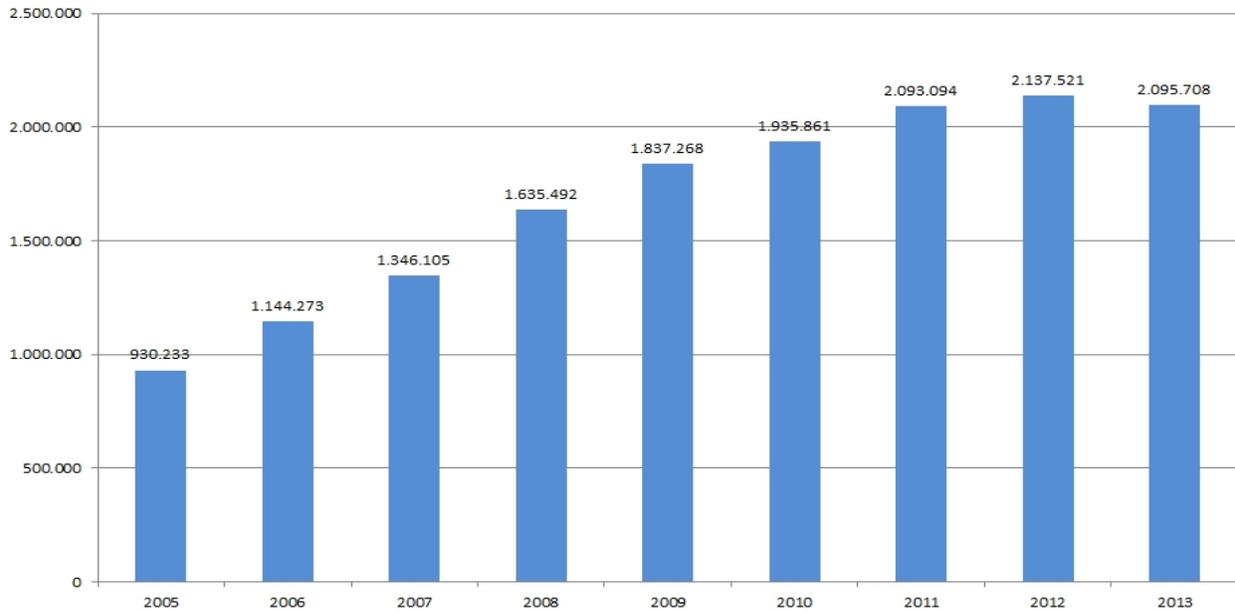
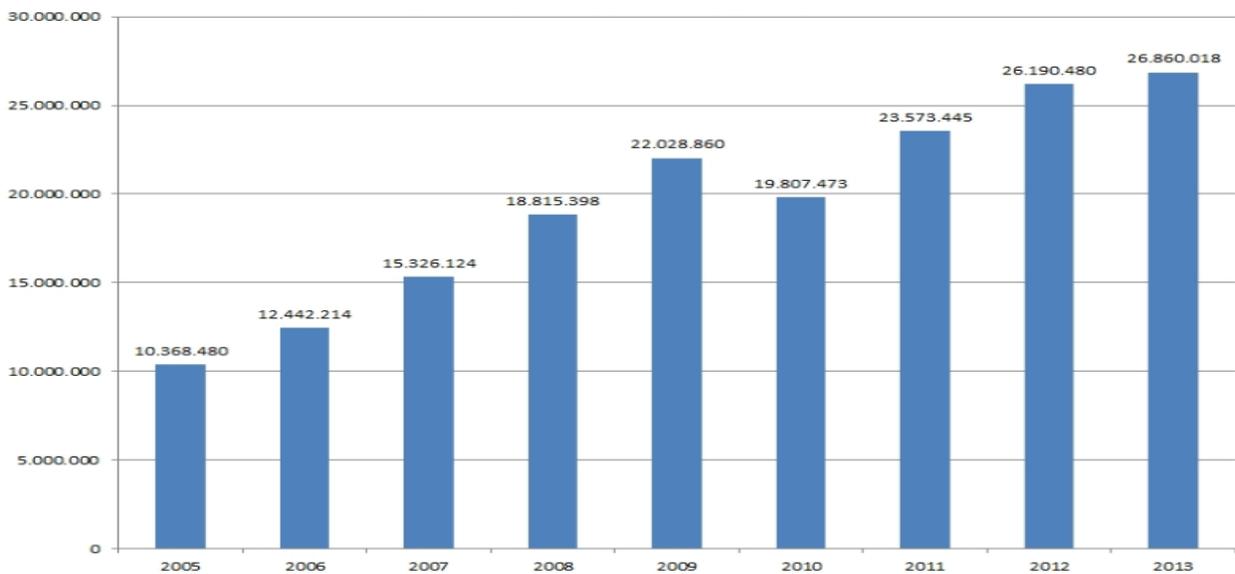


Figure 6: PPTA data on source plasma collection in the US by PPTA members**



* Data until Nov 2013

** US graph is expressed in number of collections; Data until Nov 2013

PPTA Source Plasma Collection Centers – May 2013

	North America	Europe
Fractionator-owned Centers	359	28
Independent Centers	62	55
Total	421	83

Figure 7: MRB - Type of plasma processed in Europe from 1996 to 2012 non-profit organizations (Thousand Liters)

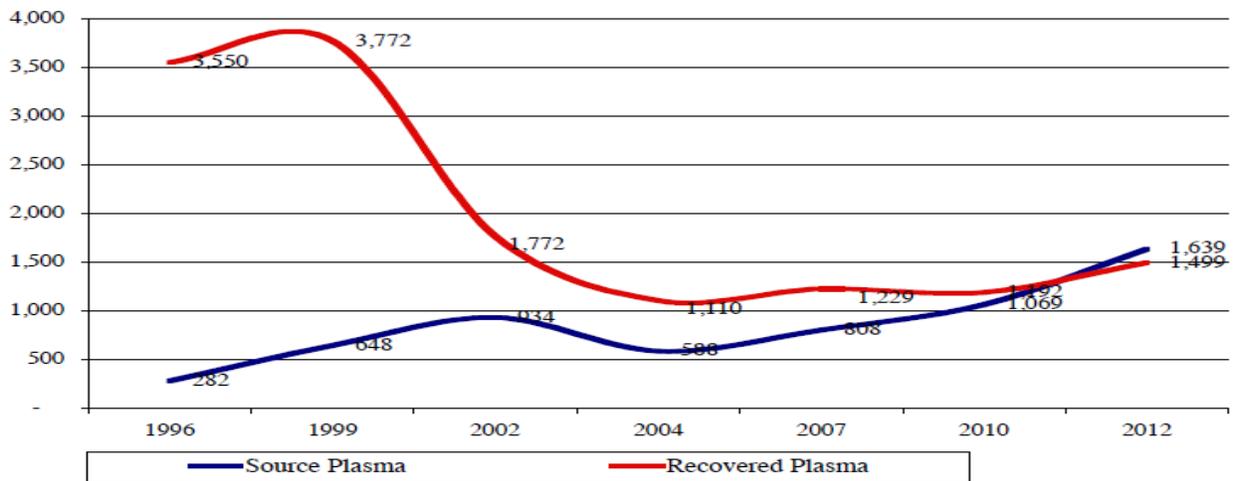
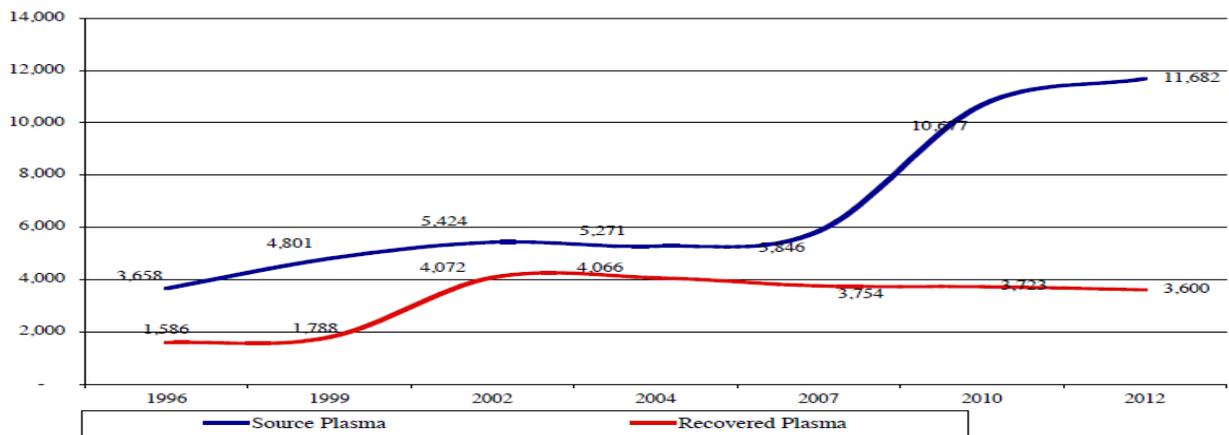


Figure 8: MRB - Type of plasma processed in Europe from 1996 to 2012 commercial companies (Thousand Liters)



INTERNAL MARKET FOR PLASMA PROTEIN THERAPIES

In Europe the plasma fractionation industry is divided into two sectors, commercial and non-for-profit (24). The non-for-profit sector in Europe was established predominantly under the auspices of the various national blood services and Red Cross societies and therefore the collection of whole blood and plasma for fractionation was an extension of the transfusion services (24). In most of these countries only the Red Cross societies can collect blood and/or plasma and only non-for-profit fractionators are allowed to fractionate plasma from these collections. Due to the close interaction between national non-for-profit fractionators and the blood collection systems, the type of plasma that is used for fractionation, comes largely from recovered plasma (1).

Over the years the number of non-for-profit manufacturers has declined. Currently, in 2014 there are only a few left: DCF-CAF in Belgium (jointly owned by the LFB (25%), Sanquin (51%) and the Belgian Red Cross (24%)), LFB in France and Sanquin in The Netherlands. In many countries the non-for-profit manufacturers stopped operating for various reasons but mostly because the costs of fractionation and investments needed to maintain the necessary quality levels exceeded the revenue that was created. This was the case for example in Finland, Denmark, Scotland, and Germany and, most recently, in England.

The commercial sector (Baxter, Biotest, BPL, CSL Behring, Grifols, Kedrion and Octapharma) is providing plasma protein therapies in most (if not all) European countries. These companies use both source plasma and recovered plasma as starting material.

In certain countries there is a preferential treatment of non-for-profit manufacturers, while dis-incentivizing the commercial sector, which affects the free movement of pharmaceutical products. At present, some Member States have implemented restrictions bereft of scientific bases and favoring national fractionators to the detriment of competitors of products within the EU (25) (26) (2) (27).

As explained, plasma for fractionation can come from both recovered and source plasma. The availability of recovered plasma is linked to the demand for whole blood. To meet the growing clinical need more plasma for fractionation is needed in future. That growth cannot come from whole blood and can only come through the collection of source plasma. It is interesting to note that the non-for-profit company, LFB, in France has acquired multiple private plasma collection centers in Austria and the Czech Republic (28), in which plasma donors are financially compensated for their time and inconvenience. This plasma is essential to meet their growing clinical need to provide more therapies in different parts of the world.

To meet clinical demand, plasma from both sources is required and companies need to make significant investments to guarantee the ongoing supply of plasma protein therapies. In that regard, the requirements are the same for all companies. It is interesting to note that there are examples of good collaboration between non-for-profit companies and commercial fractionators. Sanquin, in The Netherlands, is manufacturing a C1-esterase inhibitor product for the commercial market in the USA and has also signed a contract to fractionate a large quantity of plasma for a commercial company (29).

The starting material for plasma derived therapies is of human origin and is regulated at EU level by two pieces of legislation: the EU Blood Directive 2002/98 which regulates the collection of whole blood, blood components and plasma whatever their intended purpose is, and the EU Pharma Code (EU Directive 2001/83), which regulates pharmaceuticals including plasma protein therapies.

The EU Blood Directive is used by certain Member States to introduce national barriers which distort the EU market for plasma protein therapies. The lack of differentiation in the EU Blood Directive between whole blood for transfusion and plasma collected for fractionation has generated over the years problems of interpretation especially regarding what legislation should be applied in case of medicinal products derived from human plasma. It is not appropriate that stable medicinal products (Plasma Proteins Therapies) with a defined shelf-life are considered by some national authorities as labile products (30).

One of the objectives of the European Union is to establish an internal market where EU citizens can enjoy the fundamental freedoms guaranteed by the EU Treaties. The plasma protein therapies sector still remains fragmented and there are several national barriers which restrict patient access to therapies.

We are of the opinion that the EU must differentiate in its legislation between the collection of whole blood for transfusion and the collection of plasma for fractionation. This will reduce ambiguity in the EU law implementation and consequently prevent the introduction of national measures which, without a real public health justification, restrict the access of EU internal market benefits to patients. More efforts to increase competitiveness in Europe should be undertaken in order to ensure that patients have a stable access to therapies.

PPTA supports a system where free market competition is established. Such a system ensures not only an adequate product supply to respond promptly to changing clinical need but also will boost investments and innovation to the benefit of patients in need of these products.

SUPPORTING PATIENTS' VOICE

The ultimate goal of guaranteeing a safe and stable supply of plasma and plasma derived medicinal products is to ensure patients access to care.

As the European Commission proposed: “The position of the patient should be strengthened to achieve better and safer health outcomes. Patients need to be empowered to manage their health and their healthcare more pro-actively” (31). PPTA fully supports this and advocates that patients have to be consulted on any issue which may impact the safety, efficacy or supply of the treatment they receive.

Most of the patients treated with plasma derived medicinal products are affected by chronic rare diseases. These are caused by genetic defects which manifest themselves in early childhood and are often life-threatening.

Plasma protein therapies significantly prolong life expectancy and improve the quality of life of patients.

Life-threatening is often a characteristic related to chronic plasma-related rare disorders. Therefore, it becomes vital for patients to have continuous access to plasma-protein therapies.

The Platform of Plasma Protein Users which represents organizations of patients with treatable rare diseases linked by common therapies based on plasma derived medicinal products (PLUS), has published in 2012 its Dublin Consensus Statement on optimized supply of plasma derived medicinal products (32). Amongst their priorities, they stress the importance that adequate plasma supply should be based on identified clinical needs of patients and improved access to diagnosis (33).

PPTA strongly recommends taking into consideration patients' perspective as any reform will impact on patient access to care. Unlike in the above mentioned WHO meeting, PPTA would be pleased to see patients included in a more consistent manner in future stakeholder engagements.

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