European Haemophilia Consortium
Rue de l'Industrie/ Nijverheidsstraat 10, 1000 Brussels, Belgium
Tel. +32 (0) 2 894 23 70; Email: office@ehc.eu; Website: www.ehc.eu

EHC Round Table of Stakeholders

Tenders and Procurement of Coagulation Factor Concentrates: A European Survey of 38 countries

Meeting Report

International Press Centre

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Executive Summary

The European Haemophilia Consortium (EHC) Round Table of Stakeholders takes place three times a year and addresses issues related to the comprehensive care of haemophilia. Each event aims to explore a different aspect of comprehensive haemophilia care and European health policy, tackling common issues faced by patients as well as spotlighting the latest scientific developments.

On 15 June 2015 in Brussels, the European Haemophilia Consortium (EHC) organised its second Round Table of the year on 'Tenders and Procurement of Coagulation Factor Concentrates: A European Survey of 38 countries.' The event was an opportunity to present the findings of the 2014 survey conducted by the EHC on how coagulation factor concentrates (CFCs) are purchased in Europe. The survey also sought to identify to what extent patients and healthcare professionals are involved in the procurement of these medicines. During the event stakeholders such as patients and physicians presented their experience of involvement or non-involvement in the tender process in their countries and how this impacted the outcome of the tender. Participants also heard about considerations to take into account in the procurement of new technologies in haemophilia care and on the changes to the European Union (EU) Public Procurement Directive.

The event kicked off with a presentation from Brian O'Mahony, President of the EHC, in which he gave a quick summary of the findings of the survey. Amongst these, he noted that countries holding a tender generally purchase CFCs at a lower price. Additionally prices decrease whenever there is a legal framework and both patients and physicians are involved in the tender process.

Mr Declan Noone, Chair of the EHC Data and Economics Committee, complemented the presentation from Mr O'Mahony with details on the product selection process and economic criteria applied to product purchase. Generally criteria such as cost, quality, efficacy, safety and convenience are applied to the selection process. In addition, other elements such as tax regimens and the set-up of the distribution chain have an impact on the price of the product purchased.

Prof Paul Giangrande, Chair of the EHC Medical Advisory Group (MAG) spoke about his personal experience as a clinician in the organisation of the national CFC tenders in the United Kingdom (UK), one of the first countries to organise a national tender process for haemophilia treatment. He described how the national tender was implemented and the challenges encountered such as identifying patients willing to switch to a new product and allocating the product purchased equitably amongst the different centres.

Prof Cédric Hermans, Vice-President of the European Association for Haemophilia and Allied Disorders (EAHAD) followed with an overview of the situation in Belgium, a country that does not currently have a national tender process. As a physician, he noted that there are many opportunities for improving the current situation in which much of the haemophilia budget (approximately 80 per cent) is spent on CFCs leaving only minimal funding for comprehensive care services. Prof Hermans argued that much of this could be improved by having a central approach to haemophilia care, with a national registry, official treatment centres and a national tender.

Mr Miguel Crato, President of the EHC Portuguese National Member Organisation (NMO) gave an overview of patient involvement in tenders in Portugal. There, patients are officially involved in providing advice in the pre-tender phase, however their advice is non-binding. Mr Crato explained that currently cost is the sole criterion for selecting who is awarded the tender. The Portuguese patient organisation is currently working towards changing that and ensuring that other criteria such as safety, quality and efficacy are also included in the tender process.

Dr Edward Laane from the North Estonia Medical Centre then presented the situation in his country, Estonia. He noted that physicians have only recently become involved in the tender while patients are still not involved. He explained that the country had gone through some corruption scandals, which had considerably impacted patients' and physicians' trust in the healthcare system.

Mr Jaroslav Kracun from the European Commission presented the new EU Public Procurement Directive, which has the primary goal of opening the market to as many economic operators as possible, hence creating competition and lowering prices for services and products. The new EU legislation will have to be transposed into national legislation by April 2016 and offers new methods for procuring services and products such as the pre-commercial procurement and the innovation partnership.

Prof Flora Peyvandi, Member of the EHC MAG, detailed some considerations to be made with regard to the procurement of new technologies in haemophilia treatment that will become available to European patients in the coming years. Prof Peyvandi noted that although the benefits of these treatments were clear (i.e., prolongation of product half-life), it was important for doctors and patients to establish what treatment protocols would be best suited to which patients and in what situations (e.g., bleeds, prophylaxis, surgery).

Dr Paul Rübig, Member of the European Parliament (MEP), concluded the event with a few remarks on the importance for the European Parliament of supporting patients' access to these new technologies.

The event concluded with a Q&A session.

The main take away message of the Round Table was that the involvement of patients and physicians in the tender process not only brings invaluable benefits in terms of budget impact but can also offer considerable insight to tendering authorities on current patient needs and preferences.

Participants agreed that a centralised approach to procurement with a national haemophilia council, a national registry and a single haemophilia care budget and strategy provides the best outcome in terms of resource use and allocation and access to treatment and comprehensive care. Such a system also helps all parties involved in the provision of haemophilia services to have an accurate idea of the number of patients, the type of treatment administered, potential side effects and the effectiveness of treatment.

Participants also agreed that although longer-acting factor concentrates bring a considerable benefit with longer half-life, there is still much to be discussed on how best to allocate these treatments. Additionally, monitoring of any potential adverse events is critical.

Finally, with the new EU Public Procurement Directive, although tendering authorities will be able to have cost as the sole criterion for running a tender, other economic aspects that can be closely linked to patients' quality of life can also be taken into account, such as whether the use of a particular product or regimen will cut down on other expenses including hospitalisations and surgery. Nonetheless, all present stressed that quality, safety and efficacy are key elements to be taken into account whenever CFCs are purchased.

Meeting Report

On 15 June 2015, the European Haemophilia Consortium (EHC) hosted its second Round Table of the year at the Residence Palace International Press Centre in Brussels, Belgium. The event aimed to present and discuss the findings of the EHC survey on the procurement of coagulation factors concentrates (CFCs) published in May 2015 in the scientific journal *Haemophilia*[1].

Results from the Survey

Mr Brian O'Mahony, President of the EHC, opened the event with a presentation detailing the findings of the EHC 2014 survey on how CFCs are procured in Europe. The survey was sent to all 45 EHC members and responses were received from patient representatives and clinicians in 38 countries.

The survey quickly identified three different procurement methods: tenders (used in 19 of the responding countries and used primarily in Central and Eastern European countries), alternative methods and a combination of both. The survey also looked at the selection criteria used in each of these different methods. The results were that price and safety are the most important selection criteria followed by quality, efficacy, supply and convenience.

Plasma-derived and recombinant CFCs for haemophilia A and B were the products most widely tendered for, followed by plasma-derived factor VIII in combination with von Willebrand factor (FVIII/VWF), prothrombin complex concentrates (PCCs) and bypassing agents. Only seven countries tendered for products for other rare bleeding disorders.

This survey also established that a variety of stakeholders are involved in both tender and procurement processes. Their involvement can be either legally required or informal and some of these stakeholders may only be involved in an advisory or observer capacity. Stakeholders involved in tenders and alternative methods include: health insurance funds, medicines agencies, pharmacies, hospitals, representatives from Ministries of Health, clinicians and patient organisations.

All countries with a tender process have a legal framework in place to hold these tenders, while in countries with alternative or combined processes, only 14 out of 19 countries have a legal framework in place. Patient organisations are more involved in countries holding tenders (15 out of 19 countries involve patients), however only two countries give them a formal role. With regard to alternative and combined processes only six out of 19 countries involve patients and only one country gives them a formal role.

The survey showed that countries holding a tender purchase CFCs at lower prices. Prices are additionally lowered when there is a legal framework in place and when both patients and clinicians are involved in the tender or procurement process.

The study also revealed that a coordinated national system delivers the best outcomes, as it optimises the use of resources for haemophilia care (a single budget for a single haemophilia strategy). Moreover, the use of a registry appears to be the most effective way to predict factor use and plan factor purchase. Also, the involvement of patients and clinicians ensures more transparency and trust in the healthcare system as well as a more appropriate assessment and selection of products resulting in lower prices.

Mr O'Mahony concluded his presentation by saying that it was important to ensure that any proportion of savings made by the use of tenders be re-invested in the comprehensive care of haemophilia and other bleeding disorders.

Product Selection and Economic Aspect

Mr Declan Noone, Chair of the EHC Data and Economics Committee, proceeded to present a more detailed overview of the findings of the survey concerning different models for the CFC selection process and related economic aspects of procurement.

[1] O'Mahony, B. et al. Survey of coagulation factor concentrates tender and procurement procedures in 38 European Countries. *Haemophilia* (2015), 1-8.

He explained that there is a wide variety of methods amongst the procurement models. For instance, tenders can be organised either at national level or as a coordinated regional purchase. Tender boards and tender contracts are limited in time with tender boards having a mandate of approximately two years and tender contracts being made for one to three years on average.

With regard to the alternative methods, the selection criteria included, amongst others:

- lowest price on a national basis,
- preference of individual hospitals, states, insurances,
- national maximum price,
- all products available.

Although price is the main and often most weighted criteria for the selection of CFCs, Mr Noone emphasised that it is by no means the only criteria. As Brian O'Mahony mentioned, other elements, such as safety, efficacy, quality and convenience are taken into account in the product selection process.

The survey also indicated that 74 per cent of countries with a tender use patient registries to predict volumes against only 22 per cent of countries with alternative methods. The use of a patient registry is associated with greater accuracy of estimated CFC use in a country as it provides data on actual CFC consumption.

Besides the legal framework, patient and clinician involvement, the survey reported other elements that have an impact on prices in the tender process. These are, for example, the involvement of a tender or procurement committee, which leads to lower prices. The different tax regimens applied on CFCs whether or not contracting agents are used and the set-up of the CFC distribution chain are other factors that affect price.

Alternative procurement options also include the use of health technology assessments (HTAs) or different reimbursement schemes based on either a cost per patient, cost per treatment or cost per sustainable virologic response (SVR).

Mr Noone concluded his talk with a few notes on what the future of haemophilia procurement may look like. He referenced the new technologies in haemophilia care coming to the market in the coming years and the increase of personalised therapies based on each patient profile.

Clinical involvement in the tender process

Prof Paul Giangrande from Oxford University and chair of the EHC Medical Advisory Group (MAG) gave a presentation on his involvement as a clinician in the United Kingdom (UK) tender process.

Prof Giangrande started his talk by noting that the UK was one of the first countries to introduce a national tender for large volumes of CFCs. Although the national tender was very successful in reducing CFC prices, it did not necessarily lead to a budget decrease for haemophilia; in fact, the haemophilia budget has increased steadily over the past ten years. Prof Giangrande listed several factors that have caused the haemophilia budget to increase. One is a growing patient population, with patients coming from outside the UK. Another is that prices for bypassing agents and FIX concentrates have not decreased significantly as quantities purchased are too small for price bargaining. Finally, larger centres in the UK were already negotiating lower prices prior to the tender thanks to the large quantities purchased, so in this case the budget savings were less pronounced.

The national tender was introduced in the UK with the objective of decreasing CFC prices. However authorities also wanted to maintain the plurality of products and preserve the prescribing rights of physicians. Patient organisations and clinicians were involved throughout the process of developing the tender. Prof Giangrande noted that, thanks to this, there was no significant increase in the number of inhibitors following the implementation of the tender. Furthermore, authorities used this opportunity to

implement home treatment, which also impacted the price, since unlike medicines for hospital use, medicines for home use are not taxed.

The UK tender is led by the commercial medicines unit of the Department of Health that, while very experienced in purchasing large volume of medicines, has no specialised knowledge of haemophilia or CFCs. For this reason, a committee was set up by the UK Haemophilia Centres Doctors Organisation (UKHCDO), made up of their members along with two purchasers and a patient representative with observer status. The role of the doctors on this committee was to evaluate CFC quality, safety and efficacy and to set volume bands. They were not, however, involved in either the purchasing process or in meetings with companies.

At the beginning of the process, a series of regional consultations were organised to evaluate how to allocate product distribution in a given region. Then, physicians had to identify patients who would be good candidates for switching products, keeping this to the lowest possible number. Physicians decided not to switch the product of patients with less than 150 exposure days and patients who had recently undergone immune tolerance induction (ITI) therapy. Patients were informed about these changes through face-to-face meetings, by letters and by nurses who also provided training on how to use these new products. Monitoring systems were implemented to ensure that centres maintained their targets for annual consumption and to check that there would be no increase in inhibitor development.

Following each tender, physicians are asked to assess the performance of various companies supplying CFCs based on a series of criteria, such as timeliness and accuracy of deliveries, customer service and added-value of the services provided by the company.

Prof Giangrande concluded his presentation by highlighting certain factors to take into consideration when organising a tender, including potential conflicts of interest between clinicians and pharmaceutical companies and medico-legal liability insurance in case of problems associated with new products.

Clinical involvement in alternative processes

Prof Cédric Hermans, from the Catholic University of Louvain and Vice-President of the European Association for Haemophilia and Allied Disorders (EAHAD), described the situation in Belgium where there is currently no national tender process. Belgium has approximately 1,000 patients with haemophilia and the yearly consumption of FVIII concentrates is close to 8 million international units (IU).

In Prof Hermans' opinion, the lack of a centralised approach for haemophilia care in Belgium has led to an inefficient use of public resources. For instance, Prof Hermans believes that a centralised tender would encourage the Belgian government to put in place monitoring systems that allow for better tracking of the exact number of haemophilia patients in Belgium (currently unknown), their treatment regimens and the efficacy of these treatments. Additionally, a monitoring system for haemophilia care would give a clear overview of exactly how much money is spent and for which services. This would enable a re-balancing and re-allocation of budgets wherever necessary. For instance, Prof Hermans estimated that currently Belgium is spending nearly €100 million on haemophilia care with 80 per cent of this budget going to the reimbursement of CFCs. This results in haemophilia centres often receiving limited funding and struggling to provide comprehensive care services to their patients, including laboratory diagnosis and genetic testing, treatment of inhibitors, co-morbidities and musculoskeletal complications, and psychosocial support. Moreover, haemophilia patients still do not receive hepatitis C (HCV) treatment, which means that while extraordinary amounts of money are spent on CFCs, many patients are dying from co-morbidities. Prof Hermans noted that in his opinion this is an absurd situation.

Prof Hermans also pointed towards other elements contributing to a lack of efficiency in the management of haemophilia care in Belgium. For instance, in Belgium any physician (specialised or not) can prescribe haemophilia treatment, meaning patients can get inappropriate treatment that could result in wasteful use of CFCs. Furthermore, Belgium has a claw-back system, which means that if at the end of the year pharmaceutical companies have over-sold in one area, they need to partially reimburse the government. However, the money reimbursed is not necessarily reinvested in the area where companies have over-

sold, such as haemophilia. Also, Belgium has not implemented all ten of the 'Principles of haemophilia care' developed by EAHAD, including having a central organisation to coordinate haemophilia care, having a national patient registry and treating all patients at a comprehensive care centre or haemophilia treatment centre.

Prof Hermans argued that Belgium could apply some strategies already in place in other European countries to optimise haemophilia care funding, such as holding national tenders, introducing budget capitation (fixed reimbursement amounts per patient), centralising haemophilia care, conducting health technology assessments (HTAs) and creating a doctors' coalition (similar to the UKHCDO). By implementing these strategies, costs of haemophilia care could not only be contained but also better monitored, and budgets could be re-allocated to areas that are currently underfunded. Furthermore, he believes that clinical patient outcomes should be standardised and patient treatment should be individualised depending on the patient's medical history and lifestyle.

Prof Hermans concluded that while access to and reimbursement of CFCs is critical, it should not negatively impact access to comprehensive care and new treatments. Furthermore, he called for a re-evaluation of current cost models, such as cost per unit, and suggested new pricing methods be looked at, such as cost per year. Finally, he stressed that all stakeholders, including patients and physicians, should be involved in the discussions around the funding and organisation of haemophilia care.

Patient involvement in the tender process

Miguel Crato, President of the EHC Portuguese National Patient Organisation (NMO), outlined the current situation with regard to the purchase of CFCs in Portugal.

Although, Portugal uses a tender process to purchase CFCs, it does not have a national patient registry, which makes it more difficult to keep track of the amount of CFCs consumed and number of patients treated and to plan ahead. Whenever a tender is announced, each hospital initiates its own local tender and specific quantities of CFCs are purchased based on each hospital's previous use. It is worth noting that like in Belgium, there is no national certification for haemophilia centres and each hospital can claim to be specialised in haemophilia care. This is, however, changing thanks to the certification project of the European Haemophilia Network (EUHANET), which recognises haemophilia centres as European Haemophilia Comprehensive Care Centres and European Haemophilia Treatment Centres. So far, five centres have been certified in Portugal.

Mr Crato then went on to explain how the Portuguese tender is set up. Whenever a tender is organised, a tender commission of five people is formed. This commission includes a medical specialist, such as a haematologist or immunologist. Since 2013, the patient organisation has a formal advisory role in the preliminary stage of the tender, although their advice is not legally binding. To better carry out this advisory role, the Portuguese NMO organises a series of meetings between patients, hospitals and clinicians and between patients and the tender commission. This is to remind them of their formal role and to voice their concerns and opinions on the situation of haemophilia care based on scientific and medical information and on personal experience. Mr Crato also noted that in Portugal patients have the legal right to refuse to be switched to a different product without their formal consent. This often places the hospital and manufacturer in a difficult situation because they do not manage to use the product purchased through the tender. On that note, Mr Brian O'Mahony commented that the survey looked at this aspect and found out that in six out of the 19 of countries that carry out a national tender, patients can decide to remain on the product they were previously on.

Currently the main criterion for the selection of CFCs is price and in the past couple of years, the Portuguese NMO has been actively campaigning with the government to point out that cost should not be the sole criterion for CFC purchase. The NMO reminded the government that the HIV and HCV contamination took place at a time when cost was the sole criterion for product selection. Furthermore, in 2015 the Council of Europe issued a resolution on haemophilia care, which stresses that cost should not

be considered as the sole criterion for CFC purchase[2]. Finally, the Portuguese NMO pointed out that it makes no sense to have a clinician on the tender board if cost is the sole criterion taken into consideration. The clinician can provide important information on other criteria, such as quality, efficacy and safety, which need to be considered.

Impact of non-involvement of patient organisations

Dr Edward Laane from the North-Estonia Medical Centre gave a presentation on the situation in Estonia where patient organisations and clinicians are not involved in the tender process.

Dr Laane noted that the situation in Estonia lacks transparency. Although public tenders are organised in line with legal requirements, the country has experienced a series of troubling situations with corruption scandals and conflicts of interest between government officials and pharmaceutical companies. These events did not promote trust in the system by either patients or physicians.

In Estonia some tenders are organised for the fractionation of surplus plasma collected by hospitals. This means the hospital is forced to source its product from a single supplier based on the best price. In fact, Dr Laane noted that in terms of tender criteria, pricing is weighted at 98 per cent while pathogen inactivation techniques only count for 2 per cent of the final rating of a tender proposal.

Dr Laane cited the example of the 2010 tender process in which no clinicians or patients were involved. Following the tender clinicians were informed of the final decision by an explanatory letter from pharmaceutical companies with no opportunity to provide any comments or advice on which product to select. Since then, and after uncovering some scandals linked to a conflict of interest, the tender commission now includes a haematologist but still no patient representative. Furthermore, he noted that price continues to increase rather than decrease due to the fact that there is a quasi-monopoly of product distribution from a single company and poor management of tender contracts.

Dr Laane concluded by pointing out some of the negative impacts of non-patient involvement in the tender process, which include a loss of trust in the healthcare system, the reimbursement process and the pharmaceutical industry. His hope is for patients to be involved in the procurement process in Estonia.

Overview of the revised EU Public Procurement Directive

Mr Jaroslav Kracun, legal officer at the European Commission Directorate General for Internal Market, Industry, Entrepreneurship and SMEs (DG GROW) gave an overview of the changes in the revised Directive for Public Procurement (2014/24/EU) that will come into effect in April 2016.

Mr Kracun reminded the audience that the first objective of the Directive is to open the European market to all economic operators and that this should result in lower prices thanks to increased competition. Secondly, the Directive aims to simplify the tender process and to reduce the administrative burden. Finally, the new legislation will strongly promote e-procurement.

With this new legislation the tender can be awarded either based on targeted technical specifications or according to the evaluation of the most economically advantageous tender (MEAT), which will look at both pricing and qualitative characteristics of the product or service tendered for.

However, pricing remains a key element and tendering authorities can decide to make it the sole award criterion. The price criterion, however, is not limited to the price of a good or service but also to the cost related to the purchase, ownership and general life cycle of the product or service. Another award criterion can be the best price-quality ratio, which looks not only at the price and cost but also at other qualitative criteria relevant for the specific tender such as quality, environmental or social aspects. Another way of presenting the tender is to give bidding entities a set budget and award the tender based on the best quality of the product or services sought after. The cost can also be calculated based on cost-effectiveness or a life-cycle approach, which could be more relevant for innovative products and services.

[2] Resolution CM/Res(2015)3 on principles concerning haemophilia therapies.

Mr Kracun noted that for hospital services or products another economic criterion that could be taken into account when developing the tender is the potential cost saved from improving the patient's health (e.g., avoidance of additional hospital stays or additional medical interventions).

Life-cycle assessments should be specific to the product tendered for and have objective, verifiable and non-discriminatory criteria.

The new Directive also includes two new procedures: the pre-commercial procurement and the innovation partnership.

Pre-commercial procurement involves different suppliers competing through different phases of development. The risks and benefits are shared between the procurers and the suppliers under market conditions.

The innovation partnership allows tendering authorities to tender for a solution to a problem or situation. This would allow companies to propose tailor-made solutions to the tendering authorities' needs. In this case the tender is neither for an existing product nor service but for the development of a solution to a problem.

New technologies for haemophilia treatment

Prof Flora Peyvandi from the University of Milan and member of the EHC MAG gave an overview of issues surrounding the procurement of longer-acting CFCs (i.e., CFCs with an extended half-life compared to current treatments).

Prof Peyvandi explained that these medicines were not yet available on the European market but are expected to be introduced in the coming years. Nevertheless, she noted that it is important to look at the clinical use of these products in North America and to already identify and evaluate their benefits. It will also be necessary to see how these products compare to current treatments and how they can fit in haemophilia strategies and budgets.

Obviously, the main advantage of these new therapies will be the prolongation of the protein half-life, even though the extension is much more significant for FIX concentrates (three to six fold) than for FVIII (one and half to three fold). Half-life extension could translate into better protection for patients (with higher trough levels) and less infusions, and this is predicted to be particularly beneficial for children. Also, these products may be less immunogenic, even though clinical data is not yet available on this point.

Prof Peyvandi noted that the use of these products will differ for acute bleeds, surgery and prophylaxis and for individual patients based on their patient history, genetics and lifestyle. Patients and physicians should have a discussion on whether patients should continue to infuse as frequently as they do with current products, so that higher trough levels and protection are insured, or whether patients could infuse less often, although this may result in a greater risks of bleeds.

In conclusion, Prof Peyvandi believes that longer acting prophylactic regimens are not likely to be any cheaper than current regimens, however she hopes that the pricing can be adjusted so that patients receive the same amount of product and maintain the current frequency of infusions for greater protection of the patient.

Remarks from Dr Paul Rübig, Member of the European Parliament

Dr Paul Rübig made some concluding remarks on the event and congratulated participants for the successful meeting. He explained that it was important for Members of the European Parliament (MEPs), like himself, to learn more about best practices in health care across Europe. Dr Rübig reminded the audience of the important role of e-health technologies in the current European landscape to provide cost-effective solutions to health problems. He also stressed that small and medium enterprises play a crucial role in developing innovative treatments and technologies in the healthcare field while helping Europe to stay competitive.

Dr Rübig noted that he is the chair of the European Parliament Science and Technology Options Assessment (STOA), a scientific body that provides MEPs with scientific and technological innovations in the context of their legislative work. Dr Rübig hopes STOA will be able to share best practices for public tenders and to promote collaboration amongst Member States. One of the areas covered by the STOA is called 'perfect life,' which considers the cost-effectiveness of different kinds of technologies in healthcare.

Dr Rübig also mentioned that he and some of his fellow MEPs created a rare bleeding disorders' support group, established to help people with these types of bleeding disorders in Europe. The EHC provides information to this group with regards to advances in haemophilia and new technologies for treatment, amongst others.

Dr Rübig concluded his remarks by noting that there are many good products and treatments to improve EU citizens' lives and that it is important that they be able to access these new technologies.

Discussions

The Round Table concluded with a question and answer period. During the discussions Prof Hermans reiterated the need for reliable data on patients and their treatment, for designating official treatment centres and for organising haemophilia care with a holistic approach at national level. Mr O'Mahony noted that this can be done by implementing a national haemophilia council, which is one of the Council of Europe Resolutions for haemophilia care. Prof Hermans also hopes that if prices for CFCs can be reduced, the money saved could be re-injected into comprehensive care services.

Prof Giangrande was asked how the UK tender was organised in terms of the volume of CFCs purchased and whether in terms of safety criteria, a specific emphasis was placed on inhibitor development. Prof Giangrande explained that different volume bands were allocated to different companies. The product was then distributed amongst different centres in the same region ensuring that each centre would maintain product usage in line with national guidelines and set objectives. With regard to inhibitors, the Tender Board in 2014 concluded that all products were equally safe. However, some products were considered at higher risk for inducing inhibitors and therefore were not used on previously untreated patients (PUPs) or patients with a low number of exposure days.

There was a question regarding whether it would be possible to establish a European registry for people with bleeding disorders. Panellists replied that it seemed unlikely that this would happen in the near future. However, it was stressed that it would be very important to have a data collection system that is similar in all European regions to record the same type of data on safety and efficacy.

Prof Angelika Batorova commented on the fact that many countries in Central and Eastern Europe, like her home country, Slovakia, where she currently practices, had started haemophilia care in the 1950s and implemented systems of comprehensive care as early as 1970s. She noted that, as Mr O'Mahony mentioned in his presentation, these countries often have a national registry and a national tender process, thanks to which they have been able to gather detailed data about haemophilia care. In Prof Batorova's opinion, this was the reason why these countries were able to easily move from cryoprecipitate to factor concentrate, because they had a clear idea of the number of patients treated and of the treatment use. Prof Batorova strongly encouraged countries that do not yet have a national registry in place to work towards its implementation, as it is an incredibly useful tool to plan haemophilia care and to enable new technologies to be introduced on the market. Prof Batorova explained that this approach to haemophilia care (comprehensive care and a centralised registry) is in her opinion what allowed Slovakia to go from 1.2 IU/per capita in the early 1990s to 6.5 IU/per capita today. Mr O'Mahony responded by saying that the reason why so many Western countries do not have a national tender for haemophilia treatment is because they were assimilated to regular medicines and fell into systems that were already in place.

Mr Kracun confirmed that the new EU Public Procurement Directive allows for joint cross-border procurement. He also explained that although the Directive only regulates public institutions and organisations, there has been some discussion at Member State level on whether private operators

closely linked to the public procurement system, such as private health insurance funds, could be covered by the Directive. This is a decision that each Member State should take when transposing the Directive into national legislation based on the organisation of their own healthcare system.

One audience member asked about the use of mobile applications (apps) by patients. Mr Noone replied that in Ireland patients can monitor their treatment intake and their general condition through an app specifically designed for haemophilia patients. The way the app functions, if the patient enters that he or she had a major bleed or an important allergic reaction to the product, for example, the patient will receive a call from the treatment centre within 24 hours to check on their status. The app also helps patients to keep track of their treatment, which allows doctors to better follow up on the patient's treatment regimen. These apps can also help in collecting data on when bleeds take place, on levels of physical activity of patients and other aspects of treatment. Mr O'Mahony commented that these apps were developed by the patient organisation and that they can be particularly useful in the context of the tendering process to provide additional information on the level of treatment use, the management of products stocks and so on.

Finally, Mr O'Mahony noted that this particular survey was created specifically for the haemophilia community and at this point there has been no discussion in conducting similar surveys in other disease areas. Nonetheless, Mr O'Mahony thinks the survey could be useful for rare diseases to have an incremental cost per Quality Adjusted Life Years (QALY) applied to them. In terms of whether this model could be applied to other disease areas that should be looked at on a case-by-case basis; however no disease area has anything to lose by having patients get involved in healthcare decision-making processes. Mr O'Mahony recommends that patients from any disease area should try to be in the room with decision-makers to make an economic case for better treatment and to discuss these issues alongside clinicians. Mr O'Mahony emphasised the importance of patients building alliances with clinicians.