Quarterly European Policy Update

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European Haemophilia Consortium

Office of the National Member Organisations

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EXECUTIVE SUMMARY

EUROPEAN UNION INSTITUTIONS

The European Commission has been particularly focused on patient rights in the last three months, which was reflected by the marking of European Patients' Rights Day 2013. This focus has impacted on current EU Directives, most notably the EU Cross-Border Healthcare Directive, which was the topic of March's EHC Round Table, as well as the EU Blood Directive, the Tissues and the Cells Directive and the Medical Devices Regulation. With the EU Cross-Border Healthcare Directive nearing the October deadline for its implementation by EU Member States, discussions and debates on amendments and the possible implications of the Directive has increased with the EU institutions.

The European Parliament has also discussed the revision of the Clinical Trials and Medical Devices Directives; with clinical trials also being a major concern with the EHC given it met with the European Medicines Agency (EMA) on the current clinical trials guidelines during June.

Recent attention has also been focused on the affordability of medicines, and discussion on a policy report looking specifically at the link between migration and the increased movement of haemoglobinopathies throughout Europe. The European Parliament has also hosted a number of important events on the prevalence of haemoglobinopathies as well as an event on newborn screening.

In the European Council, Ministers of Health have discussed the Commission's proposal on a Clinical Trials Regulation, which was recently adopted by the European Parliament's leading Committee on Environment, Public Health and Food Safety (ENVI).

The Council of Europe's European Directorate for the Quality of Healthcare and Medicines (EDQM) has published a questionnaire to allow blood donation centres to assess their blood safety management data. This reflects a growing concern to improve blood management and establish good practices.

At present, Germany is deliberating on the suitability of blood donors and whether there should be a wider acceptance of those who have riskier sexual behaviour, particularly due to the current shortage of blood donors within the country.

Lastly, the structure of the EMA is currently undergoing changes, which are expected to take full effect from the beginning of August 2013, and take approximately 18 months to complete. Given the increased dialogue between the EMA and the EHC, this will be monitored going forward.

European Commission:
Commissioner Borg on Patient Rights' Day

In April 2013, the European Commissioner for Health and Consumer Policy, Dr. Tonio Borg, published a short press release to commemorate European Patients' Rights Day (18 April 2013).

Dr. Borg's highlighted the European Charter of Patients' Rights and how it makes health a basic human right, stating that the European Patients' Rights Day 2013 should emphasise the right of patients to travel to another EU Member State for medical treatment. This right is contained in the EU Cross-Border Healthcare Directive, which was adopted in 2011 and requires implementation into national legislation before October 25th this year.

Of particular relevance was Dr. Borg's mention of 'access to specialized treatment' and 'informed choice' . Although rare diseases were not mentioned, access to specialized treatment is one of the reasons why the Cross-Border Healthcare Directive is significant for patients with rare diseases. Furthermore, Dr. Borg highlighted the importance of 'informed choice', which entitles patients to receive full access to information about possible treatment and the reimbursement for treatment received abroad. It is important to note that this is the first time informed choice has been introduced in the Cross-Border Healthcare Directive.

The press release is particularly relevant for the EHC given that the Cross-Border Healthcare Directive could have a significant impact on cross-border haemophilia treatments and standards of care in Europe. It is interesting to see that the European Commission is once again focusing on cross-border healthcare now that the implementation date is closing in. It is expected that attention from national media and national institutions will increase as the Cross-Border Healthcare implementation deadline nears, for instance, the UK's consultation on this Directive.

European Commission:
Debate in Parliament on Cross-Border Healthcare

In April 2013, the European Commissioner for Health and Consumer Policy, Dr. Tonio Borg, visited the European Parliament's Committee on Internal Market and Consumer Protection (IMCO), which saw several Members of the European Parliament (MEPs) address a number of health dossiers including the Cross-Border Healthcare Directive.

Dr. Borg highlighted the developments that have occurred in consumer policy, which shares similar developments to those taking place in health policy, such as the increased focus on product safety, market surveillance, product traceability, and providing greater clarification of consumer rights.

Mr António Fernando Correia de Campos (S&D, Portugal), asked the Commission for an update on the implementation of the Cross-Border Healthcare Directive and received a reply from Commissioner Borg stating that the process is going well and the Directive is on track to be implemented. However, some problems are expected to arise as the implementation date

draws nearer, as there is the possibility that certain Member States may refuse prior authorisation.

European Commission:
Article Emphasises High Quality of Blood in Cross-Border Care

In May 2013, the European Commission released an article for European Patients' Rights Day, which highlighted 10 benefits for patients living in the EU. The majority of these benefits are related to cross-border healthcare.

The article highlighted how patients have the right to benefit from high quality standards for blood, organs, tissues and cells. The is relevant for the EHC as it not only incorporates patients' rights, but has the potential to impact on the upcoming revision of the Blood Directive, as it emphasises blood and plasma safety in the EU. The article presents an opportunity to emphasise patient rights and patient choice within the discussion on the Blood Directive revision.

The article highlights the importance of the 10 patient benefits being incorporated within the EU Blood Directive, Cross-Border Healthcare Directive, the Tissues and Cells Directive and the Medical Devices Regulation.

Other benefits incorporated within the list include the right to be treated with safe and effective medicines, to be treated by qualified healthcare professionals as well as to receive information on safety and quality standards in EU countries.

Also included in the article was the Commission's position on the 7th European Patients' Rights Day event, which took place on the 16 May 2013, in the European Social and Economic Committee. The event was attended by a number of patient's organisations, healthcare professionals' associations, as well as European Commission representatives.

The event focused predominantly on the implementation of the Cross-Border Healthcare Directive and the difficulties for patient organisations to be consulted within this process. It also revealed complications currently faced by a number of Member States. The Commission recognised the need for patients' organisations to be involved in monitoring and providing feedback, which could present an opportunity for the EHC to raise awareness on rare blood disorders.

European Commission:
Entry by Commissioner Borg on Thalassaemia

On World Thalassaemia Day (8 May 2013), the European Commissioner for Health and Consumer Policy, Dr. Tonio Borg, published an article on Thalassaemia, a rare genetic blood disorder, which causes red blood cells to be weakened and destroyed. Patients suffer from symptoms such as iron overload, shortness of breath, jaundice, poor growth, heart and liver problems and bone deformation. The article is relevant to the EHC as it demonstrates the

Commission's continued interest in rare blood disorders and suggests a personal interest by the Commissioner in rare blood disorders.

The Commissioner clearly demonstrated his support for European and international organisations, research networks, platforms of experts, as well as patient groups, who work on Thalassaemia. Significantly, he raised the importance for greater investment to be made in technology and medicine research and training programmes that will assist the clinical community and fund-raising campaigns to alleviate the symptoms suffered by Thalassaemia patients.

European Commission:
Report on Implementation of the Paediatric Regulation

In June 2013, the European Commission published a report on the "experience acquired as a result of the application of Regulation nº1901/2006 on medicinal products for paediatric use".

The report is highly relevant for the EHC as many haemophilia patients are children, who inherently have different treatment needs and standards compared to adults.

The report highlights the main achievements of the Regulation as well as the lessons that have been learnt, which subsequently revealed a conflict of interest between the Paediatrics Regulation and the Orphan Medicinal Products Regulation. It is important to note that there are currently problems with the off-label use of medicinal products for children, resulting from a lack of guidelines that state the correct dosage. The report also revealed that the Regulation will be updated by the Commission based on the current implementation, which the European Medicines Agency approves. A second report on the implementation will be published in 2017.

European Commission and Spanish Ministry of Health:
Event on Blood Transfusion

In June 2013, the Executive Agency for Health and Consumers of the European Commission held a meeting in Madrid on Transplantation and Blood Transfusion, in partnership with the Spanish Ministry of Health, Social Services and Equality.

The purpose of the meeting was to provide an update on EU projects related to substances of human origin and the results achieved. The meeting was well attended with the majority of Commission officials responsible for policies on blood transfusion participating in the discussion.

It is important to note the attendance of the Red Cross, national blood banks such as the Dutch Sanquin, the Council of Europe's EDQM and the French Agency for the Safety of Medicines (ANSM). The outcome from the meeting sessions is expected shortly.

European Parliament:
Event on the Prevalence of Haemoglobinopathies

In June 2013, two Members of the European Parliament (MEPs) held an event called 'Haemoglobinopathies on the Move: is Europe Ready?' The event was hosted by Ms Antigoni Papadopoulou (S&D, Cyprus) and Ms Marina Yannakoudakis (ECR, UK). The event was organised by the European Network for Rare and Congenital Anaemia's, the Thalassamia International Federation, the International Organisation for Migration and was supported by Novartis Pharma.

The meeting focused on a policy report that looks specifically at the link between migration and the increased movement of haemoglobinopathies through Europe, such as Sickle Cell Disease and Thalassamia. The report was published by the above stated organisations. The report provides policy recommendations which encourage greater recognition of the problem faced by immigrants, an improvement in data collection, to reduce the differentiation in treatment offered throughout Europe, to increase awareness of the problem, and to boost the training of professionals in dealing with the spread of haemoglobinopathies.

The report is relevant for the EHC as both sponsoring MEPs share a clear interest in the blood disorders, which is important to keep in mind when initiating advocacy programmes in the European Parliament. Lastly, it is significant to note that these diseases are treated with blood derived products.

European Parliament:
Event on Newborn Screening

In April 2013, a workshop on newborn infant health was held in the European Parliament, hosted by the Health Working Group of the Environment, Public Health and Food Safety (ENVI) Committee.

Such workshops organised by the Health Working Groups are usually well attended by MEPs, who use them to stay updated on topics on the current agenda and which are likely to impact on their work.

The workshop highlighted the problem of premature birth as well as infant and mother mortality in the EU. The discussion focused primarily on:

- Healthcare consequences of preterm birth.
- Prevention during and before pregnancy.
- Availability, quality and access to Newborn Intensive Care Units.
- Preterm birth and its consequences for chronic diseases later in life.

European Parliament:
Draft Report and Opinions on Medical Devices Regulations are Debated

In April 2013, a draft report was published on the proposal for a Regulation on medical devices, which was delivered by Rapporteur Ms Dagmar Roth-Behrendt (S&D, Germany). The draft report was debated in the Environment, Public Health and Food Safety (ENVI) Committee.

The report is of interest to the EHC as it presents one of the key health legislations being discussed in the European Parliament during its current term. Furthermore, the report runs parallel to the revision of the Clinical Trials Regulation proposal. These two dossiers continue to occupy the health Members of the European Parliament (MEPs) and will do so for the continuation of the year.

Both the report and debate focused on the Rapporteur's wish to change the Commission's proposal by creating a centralised marketing authorisation for devices. This suggestion is similar to the central authorisation procedure for medicinal products, which is currently in place under the European Medicines Agency (EMA). However, the focal point of the debate was on whether the system for medicinal products would be appropriate for medical devices, a suggestion which divided MEP opinion.

Ms Roth-Behrendt also introduced changes to the wording outlining the reprocessing of single-use medical devices, thereby removing the wording proposed by the Commission. In addition to this, her intension to make all devices reusable by default was also set out.

Finally, she intends to improve the function of Notified Bodies, (who are responsible for carrying out the assessment of medical devices) by increasing transparency, a theme which runs throughout her proposed amendments.

European Parliament:
Article by MEPs on the Affordability of Medicines

In May 2013, an article was published on Euractiv, contributed by the following Members of the European Parliament; Alejandro Cercas MEP (S&D, Spain), Nikos Chrysogelos MEP (Greens/EFA, Greece), Minodora Cliveti MEP (S&D, Romania), Marian Harkin MEP (ALDE, Ireland) and Maria do Céu Patrão Neves MEP (EPP, Portugal). The MEPs raised concerns surrounding the affordability of medicines in Europe and the impact the economic crisis is having upon on the sustainability of European healthcare systems.

The article is significant to the EHC as it feeds into the general debate on the cost of healthcare in Europe, particularly because it suggests that cost of healthcare is high on the political agenda in the European Parliament. Furthermore, it is likely that the focus on the cost of healthcare will lead to greater attention on the pricing and affordability of medicinal products in the European Union and the need to introduce reference pricing. The most important statements can be summarised as follows:

Alejandro Cercas MEP (S&D, Spain) stated that it is unacceptable that billions of Euros are spent on research for medicines, which are too expensive for patients to afford.

Nikos Chrysogelos MEP (Greens/EFA, Greece) addressed the high number of Europeans who lack access to treatment, in a system that is failing.

Minodora Cliveti MEP (S&D, Romania) raised concerns about the lack of medicine in her country, and the many hospitals closures that have occurred.

Marian Harkin MEP (ALDE, Ireland) commented on the high price of medicines in her country compared to the other Member States.

Maria do Céu Patrão Neves MEP (EPP, Portugal) mentioned that the research and innovation sector is likely to be the only area to remain unaffected from recent EU budget cuts.

European Union:
MEP Dr. Buşoi Becomes the New President of the National Insurance House in Romania

In June 2013, Dr. Cristian Buşoi MEP (ALDE, Romania) was appointed as president of the National Health Insurance House (NHIH) in Romania. This new appointment is relevant to the EHC as Dr. Buşoi is strong supporter of rare disease organisations, particularly rare blood disorders.

This is the first administrative appointment for Dr. Buşoi, managing a body which is responsible for around 80% of Romania's public health funds.

Council of the European Union:
Views on Clinical Trials

In June 2013, the Ministers of Health discussed the Commission's proposal on a Clinical Trials Regulation, which was recently adopted by the European Parliament's leading Committee on Environment, Public Health and Food Safety (ENVI).

The proposal is relevant for the EHC as the main incentive to revise the current legislation is to improve clinical trials for rare disease patients, which has the potential to impact on the treatment of haemophilia patients.

The reaction by the Working Party on Pharmaceuticals and Medical Devices on the Commission's proposal is generally positive, however some reservations are held by a number of Member States. Reservations are currently held on the short time frame that is provided for the authorisation of a clinical trial and the need for the Regulation to make direct reference to the Ethics Committees.

Discussions between the Parliament (ENVI Rapporteur and Shadow Rapporteurs), Council and Commission are expected to commence from September with a date to be confirmed.

European Directorate for the Quality of Healthcare and Medicines: Self-Assessment for Blood Safety Management

In May 2013, the Council of Europe's European Directorate for the Quality of Healthcare and Medicines (EDQM) published a questionnaire to allow blood donation centres to assess their blood safety management. The questionnaire is the result of previous consultations with Member States. A report detailing the study results is expected to be published by the Council of Europe later in 2013.

The group working on Blood Management Supply intends to develop the evaluation methods to improve the self-assessment questionnaire, which in turn can become a tool to establish good practices in blood safety management. The questionnaire focuses on whole blood and red blood cells, but also refers generally to blood components.

This development is interesting for the EHC as it could provide an indication of future supply problems globally. The EDQM's activities are important to monitor as they are the most knowledgeable institution in Europe for blood and blood components.

European Medicines Agency: Reorganisation Plans

In May 2013, the European Medicines Agency (EMA) announced its planned reorganisation in order to ensure the agency is "fit for the future". The changes focus on three key elements:

Firstly, to make changes to improve the efficiency and effectiveness of the body, which will provide better support to the scientific work of the EMA Committees.

Secondly, to increase the sharing of data with partners and stakeholders, particularly as the EMA is becoming a central body for data and knowledge for the European medicines network.

Thirdly, the reorganisation should improve the way in which the Agency supports and meets the needs of its stakeholders and partners, particularly in the research and development of new medicines and the communication between patients and healthcare professionals.

The new organisational structure is expected to take effect from the beginning of August 2013 and will take approximately 18 months to finalise. In the meantime, the EMA will ensure the continuation of services for medicine evaluation and supervision.

This is important given the meeting that the EHC had with the EMA in June on the current clinical trials guidelines and its continuing dialogue on the issue.

European Medicines Agency:
Draft Policy on Access to Clinical Trial Data Published for Consultation

The European Medicines Agency (EMA) has published a draft policy document on the publication and access to clinical trial data for public consultation. Given that the EHC is interested in the dossier of clinical trials, the consultation is important to be aware of.

Within the consultation the EMA has set out three categories for clinical trial data and the accessibility of this data, depending on the trial and medicine. Significantly, the EMA has already begun the implementation of the changes within its system despite the finalisation EU Regulation on Clinical Trials still pending.

WORLD HEALTH ORGANISATION

Meeting on the Essential Medicines List

In April 2013, The WHO held its 19th Expert Committee on the Selection and Use of Essential Medicines. Significantly for the EHC, the possibility of adding blood (whole blood as well as red cells) onto the list of Essential Medicines was discussed.

The updated list is expected to be published in the third quarter of 2013.

World Blood Donor Day Focuses on Voluntary Donation

In June 2013, the 10th World Blood Donor Day was celebrated, which raised awareness of safe blood for transfusion and to highlight the importance of blood donation.

The WHO has now called for all countries to obtain 100% of their supplies of blood and blood products from voluntary unpaid donors by 2020, which reflects the WHO longstanding support of Voluntary Non-Remunerated Donation (VNRD). The WHO emphasises that voluntary unpaid blood donors provides the safest source of blood as there are fewer blood borne infections among this group, in contrast to donors who give blood in exchange for money.

It is interesting to note that the WHO has offered further policy guidance and technical assistance to countries that are developing national blood systems based on VNRD principles, recognising that the need for blood and blood products is increasing every year.

The Council of Europe also contributed to marking World Blood Donor day by emphasising the importance of blood donation and the need to increase awareness for safe and high quality blood. This has become a key priority within the Council of Europe's healthcare policy.

OTHER RELEVANT TOPICS

Germany:
Newspapers Report on Blood Donation Criteria

In June 2013, a number of newspapers (Die Welt, Ärtzeblatt, Handelsblatt and Focus) published articles on an amendment that will impact on the regulation of blood donations and the criteria used for the selection of donors. The papers focused particularly on men who have sex with men (MSM) and raised concerns on the current exclusion of homosexuals from donating.

The German Medical Association (Bundesärztekammer) has stated that the ban on blood donations for MSM should be loosened and intends to work towards an amendment within the EU legal framework that prevents the exemption of donors who have risky sexual behaviour.

The German Medical Association and the Federal Ministry of Health have initiated discussions to address the exclusion of MSM, which has received the support of Health Minister Daniel Bahr (Liberals), who has welcomed the move by the medical profession to seek clarification on the de facto exclusion of MSM. He also raised concern on the shortage of donors and the need for more donors to come forward.

It is worth noting that these discussions have taken place just months after the Council of Europe adopted a Resolution on the sexual behaviour of blood donors, which impacts on transfusion safety and supported the exclusion of MSM to donate. It will therefore be interesting to see if this topic receives similar reactions across the rest of Europe.

OVERVIEW OF INSTITUTIONS AND DECISION-MAKING

The European Commission:

The European Commission represents and upholds the interests of the European Union (EU) as a whole. It drafts proposals for new European laws. It manages the day-to-day business of implementing EU policies and spending EU funds.

The European Parliament:

The European Parliament is comprised of members who are directly elected by EU voters every 5 years, with the sole role of representing the people. The European Parliament is one

of the EU's main law-making institutions, along with the Council of the European Union ('the Council', also known as the 'Council of Ministers').

The European Parliament has three main roles:

1. Debating and passing European laws, with the Council;
2. Scrutinising other EU institutions, particularly the Commission, to make sure they are working democratically;
3. Debating and adopting the EU's budget, with the Council.

The Council of the European Union:

The Council of the European Union, also known as the 'Council of Ministers' or just 'the Council' should not be confused either with the European Council or the Council of Europe (see below).

The Council comprises ministers from each Member State with responsibility for the policy area under discussion. As such, the Council is not a body that has a fixed membership; rather it is a legislative concept that is given expression at any given time in one of nine distinct 'Councils'.

During a Member State's presidency, the ministerial representatives of that Member State chair the Council meetings.

Presidency of the Council gives a Member State substantial influence over the conduct of EU business during the six months of its presidency (which runs from January to June and from June to December each year).

1. The presidency represents the Council in dealings with outside bodies, including other EU bodies;
2. The presidency and the Council's general secretariat (the Council's own civil service) set the agendas of meetings, the terms of meetings, and the frequency and location of meetings;
3. The presidency has responsibility for launching and building consensuses on initiatives, i.e. it takes the lead in promoting negotiations and agreement.

The European Council:

The European Council sets the EU's overall political direction, but has no powers to pass laws. Led by its President, currently Herman Van Rompuy, and comprising national heads of state or government and the President of the Commission, it meets for a few days at a time at least every six months.

European Council meetings are essentially summits where EU leaders meet to decide on broad political priorities and major initiatives. Typically, there are around four meetings a year, chaired by a permanent president.

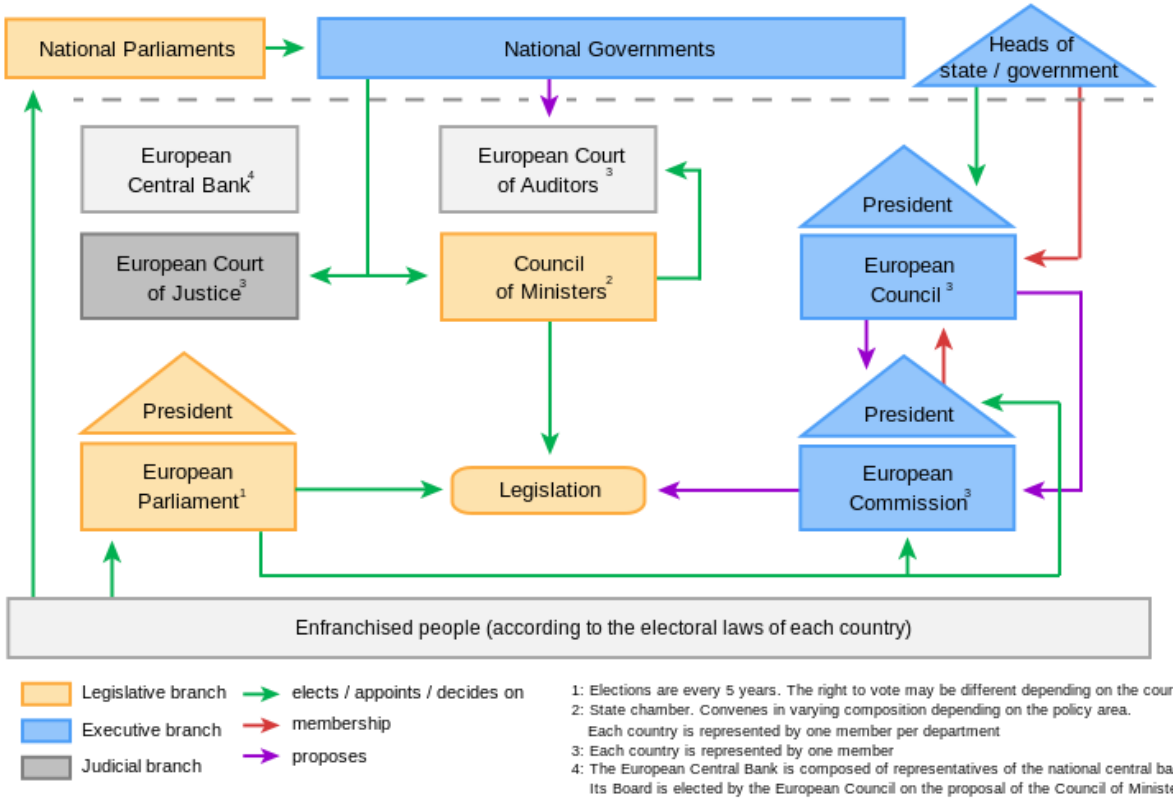
The Council of Europe:

The Council of Europe is not part of the EU, although every EU Member State is a member. Founded in 1949, the Council of Europe focuses on democracy, human rights and the rule of law. It has 47 member states (20 more than the EU) and works through the European Court of Human Rights, its main court.

The Council of Europe has a Secretary General, but not a President. It also, like the EU, has a Parliamentary Assembly which, unlike the European Parliament, is not directly elected, but is made up of members of the parliaments of its member states, their numbers (similarly to the European Parliament) based upon the population of the member state in question. The Council of Europe also has a Congress, as well as a Committee of Ministers and a Commissioner for Human Rights (the European Union does not have a Commissioner for Human Rights).

World Health Organisation:

The World Health Organisation (WHO) is the directing and coordinating authority for health within the United Nations system. It is responsible for providing leadership on global health matters, shaping the health research agenda, setting norms and standards, articulating evidence-based policy options, providing technical support to countries and monitoring and assessing health trends



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