



Meeting between the Common Representation of SoHO's associations and DG SANTE

14 March 2017

Summary Minutes

This meeting was organised in response to a request from the Common Representation of SoHO associations (CRSoHO) that brings together 4 scientific associations:

- European Association of Tissue Banks (EATB);
- European Society for Blood and Marrow Transplantation (EBMT);
- European Eye Bank Association (EEBA);
- European Blood Alliance (EBA).

The meeting opened with an introduction of the consortium. The joint members of CRSoHO represent about 60% of tissue establishments and over 90% of blood establishments in the EU. These establishments also develop and provide starting materials for Plasma Derived Medicinal Products (PDMPs) and Advanced Therapy Medicinal Products (ATMPs).

CRSoHO is committed to ensuring that substances of human origin (SoHO) activities in EU member states are governed by common principles of:

- Not-for-profit/non-financial gain;
- Voluntary and altruistic donation;
- Sufficiency and
- Sustainable pricing facilitating patient access to current and future therapies.

The different sectors involved in the consortium are regulated under a common EU legal framework and, in that context, the main goal of CRSoHO is to represent the sectors in any discussions on the development and/or implementation of that legislation. In particular, CRSoHO aims to provide expertise and input to the ongoing Evaluation of blood, tissues and cells legislation¹. The majority of members are working in tissue establishments, academic GMP-facilities and in some cases, collaborating with industrial partners in developing and delivering innovative new therapies.

The meeting was intended to introduce the consortium and its objectives to DG SANTE. The key concerns of the Common Representation were expressed. They consider that:

- The principles of voluntary, altruistic donation and of non-financial gain that apply to SoHO are under pressure from increased involvement of commercial actors. They consider it important that a principle of financial neutrality also applies on the steps following donation in order to avoid conflict of interest and possible exploitation of the donor.
- The sectors are subject to continuous innovation, improving transplant and transfusion therapies for citizens. Regulation has to follow these developments and needs to ensure a flexible and proportionate approach, in particular when collecting clinical data to demonstrate a favourable benefit: risk ratio for innovations. SANTE services confirmed this view and underlined that overall objective of

¹ Ref to evaluation/SANTE website

the regulation is to balance protection of patients with access to SoHO, including innovative SoHO therapies..

- CRSoHO expressed the view that a revision of blood, tissue and cell legislation is necessary. One of the key points to address should be a clarification of borderlines between EU legislations on blood, tissues, cells, organs, medicinal products and medical devices. SANTE services acknowledged that indeed an increasing number of innovative therapies no longer fit to the historical classifications foreseen in EU legislation but stressed that the medicinal product and medical device legislation was not within the scope of the ongoing Evaluation. SANTE services also highlighted a need to have a better overview on new technologies and innovations, and expressed hope that stakeholders like CRSoHO could provide that input.

- CRSoHO expressed their view that clarification and streamlining of the ATMP hospital exemption and its application in different Member States is required. CRSoHO pointed to the risk of non-availability of therapies for citizens without such aligning possible regimes for ATMP at EU and national level.

These points of concern are reflected in a CRSoHO position paper, which is attached to these minutes.

CRSoHO explained that several of its associations collect real world data in data registries. These registries collect and publish data on the clinical outcome of transplant/transfusion therapies, helping to ensure that patients are treated with the optimal product. The data registries are a key source of research for the professional communities. While there are overall high levels of voluntary data reporting, their view was that consideration should be given making clinical outcome reporting mandatory in the legislation.

SANTE services agreed that it is valuable to organize such data sharing platforms at EU-level, in order to acquire a critical mass of data, and allow developing and sharing knowledge in all EU Member States. Such data could also be valuable for the work of (safety and quality-) regulators and payers of HTA-bodies (health technology assessment).

CRSoHO expressed some concern on recent practices in US and Korea to fast-track highly innovative therapies. While the value and lack of alternative therapies might justify this, it remains very important to ensure safety, quality and efficacy for the recipients.

CRSoHO mentioned other key success factors to develop the sectors relating to training of doctors and nurses, availability of good practices/guidelines, open sharing of information on new products and the possibility to reduce fees and administrative costs for the professional/academic actors.

SANTE services invited CRSoHO and its members to provide inputs and express ideas in the context of the upcoming public consultation in the evaluation of blood, tissues and cells legislation.



Common representation of SoHO's Associations within Official Institutions of European Union

Meeting DG SANTE

14th March 2017

The need for representation of stakeholders *before European Union bodies* was identified by four (scientific) associations:

- European Association of Tissue Banks (EATB);
- European Society for Blood and Marrow Transplantation (EBMT);
- European Eye Bank Association (EEBA);
- European Blood Alliance (EBA).

All four associations are committed to ensuring that substances of human origin (SoHO) activities in EU member states are governed by the same principles of:

- Not-for-profit/non-financial gain;
- Voluntary and altruistic donation;
- Sufficiency
- Sustainable pricing facilitating patient access to current and future therapies

The main goal of this consortium is to provide expert opinion and supporting data to European Union decision-makers and their respective organisations in the field of SoHO. In particular, the associations wish to actively contribute to all legal and regulatory discussions affecting the SoHO field as technical experts working in the field with direct access to the experience and opinions of our members, the majority of whom are working in tissue establishments, academic GMP-facilities and in some cases, collaborating with industrial partners in developing and delivering innovative new therapies.

In support of the principles above mentioned, this *common representation* has identified the following mutual concerns:

a. Recognize the special character of SoHO's therapies:

The increasing commercialization of human substances based on for-profit businesses (against WHO *Guiding Principles on Human Cells, Tissue and Organ Transplantation* and the *EU Charter Fundamental Rights of EU²*), has a potential impact on the exploitation of donors and patients. There is a perception that the principles of non-financial gain, voluntary and altruistic donation associated with donation are threatened and include potential risk to ensuring the quality and safety of T&C and the economic impact on public health systems and patients' access to care;

b. Promote the development and access to new T&C therapies:

Changes that do not represent a substantial manipulation and do not modify the traditional transplant methods (under EC Directive 2004/23 definitions and requirements), are the result of the natural technical evolution of TE's activities, and have high potential to improve the quality and efficacy of treatment provided to patients.

T&C professionals therefore require:

² Charter of Fundamental Rights of The European Union (2000/C 364/01), Article 3: "The prohibition on making the human body and its parts as such a source of financial gain"



- Clear definition of criteria for the classification of such innovations under the EC Directive 2004/23 definitions;
 - The definition of good practices and regulation focused clinical studies, quality controls and clinical practices, applied to T&C activities with the aim of protecting patients;
 - Measures that promotes the access of patients to innovative T&C products and therapies that emerge from publicly-funded hospitals and academia as result of the needs identified by clinicians;
 - Definition of clear rules protecting the principles of altruistic and unpaid donation (defined in the EC Directive 2004/23) and patients' access to care should be in place.
- c. Need for an update of current applicable regulation, in order to:
- Ensure coherence of the legal contents and requirements of Directive on Organ donation 2010/45/EU of the European Parliament with the T&C Directives;
 - Clarify borderline products between T&C and ATMPs, as well as how to manage differences on the criteria for classification.
 - Ensure the compliance of technical aspects associated with donation for T&C, also in the context of starting materials for production of ATMPs, once Regulation (EC) N° 1394/2007 is defining that donor selection is covered by EC Directive 2004/23.
- d. Encourage procedures' harmonization regarding Hospital Exemption performed by different member states, and promote the access of patients to innovative therapies.

Changes made to processes for delivering established T&C therapies (regulated under EC Directive 2004/23) can result in reclassification as an ATMP (under the Regulation (EC) N° 1394/2007). This is a very sensitive area as reclassification as an ATMP leads to the need for a marketing authorization (MA) with significant investments of time and money without necessarily adding therapeutic value, and in the worst case scenario, potentially depriving patients of treatment.

The Hospital Exemption (HE), when well executed, can help give access for patients in individual MS to novel therapies especially when there are small target populations. HEs are awarded by national competent authorities and are subject to GMP and associated monitoring and controls. The complication lies in the variability in how it is administered between MS, and the lack of a unique registry of exemptions across the EU. Currently HE administration appears opaque and also restricts treatments to patients in that particular MS. The current system does not facilitate sharing knowledge and learning between SoHO professionals across the EU. In addition, manufacturing of products under HE has to cease once a product with MA is available. Experience with MA shows that this has not functioned as planned with most of the ATMPs approved to date subsequently being withdrawn leaving a vacuum for patients – no HE-developed product and no commercial alternative. This situation has led to frustration within the professional community with the difficulty to translate new therapies to patient benefit.