The European group for Blood and bone Marrow Transplantation (EBMT) is the largest professional organization active in Europe in the field of Hematopoietic Stem Cell Transplantation (HSCT) and related therapeutic approaches. EBMT has established links with national societies active in the same field all over European countries. EBMT also works closely with other international societies in the world that together recently celebrated the 1 millionth HSCT.

The EBMT mission and vision cover the development of HSCT through various means:

1) The analysis of HSCT results in various types of diseases that is made possible by the systematic and prospective collection of results in one of the largest registries available in the world, with clinical data from almost 377,000 patients.
2) The promotion of clinical and translational research in the field of HSCT, encompassing the development of new forms of cellular therapies, the exploration of new indications and new strategies and the improvement in clinical and biological monitoring in transplant patients in order to better predict outcome
3) The promotion of international standards for quality management in transplantation and the associated accreditation programme - JACIE (Joint Accreditation Committee - ISCT & EBMT) whose goal is to promote excellence and harmonization in the field of HSCT. The standards cover the three major fields of activities involved in HSCT, namely clinical, collection and processing, as well as their interactions. There is preliminary evidence that implementation of JACIE contributes to improving outcome of transplant patients. The Ex-post evaluation of the Public Health Programme (PHP) 2003-2008 praised JACIE highly.

EBMT is closely monitoring the implementation of the regulation on Advanced Therapy Medicinal Products (ATMPs) since it was announced in 2007. While cell products that are most often used for autologous or allogeneic HSCT do not fall into this category, one of EBMT's and its affiliated centres' goals is to establish a better definition and characterization of infused cell products in order to better control their therapeutic efficacy as well as the occurrence of side-effects; when successful, this approach may produce a situation in which these improved cell products will fall under the definition of ATMPs.

EBMT welcomes the contribution of biotechnology and pharmaceutical industry in the development of this new class of therapeutic products, and realizes the potential for economic development associated with these new activities. EBMT deeply hopes that it will speed up the process to develop more efficient therapies. However, EBMT is also concerned by the following consequences of the 2007 regulation:

1) The number of ATMPs that have received marketing authorization from the EMA since 2007 is dismal, and little affects the practice of haematology and oncology, the two main disciplinary fields in which HSCT are being used.
2) The role of academic centres in the development of ATMPs will be essential in the short- and middle term. Conditions in which the production of certain ATMPs initially designed, produced and validated by academic facilities will transition to the industry must be better defined.
3) While previous issues are far from being resolved, the deployment of ATMPs regulation in EU member and other states already imposes new, increased and costly constraints on academic facilities that traditionally deliver autologous and allogeneic minimally-manipulated hematopoietic stem cell grafts, and that additionally work to "bridge the gap" and develop new and improved cell and gene therapy processes to be evaluated in clinical trials or in orphan situations.
4) Although the regulation includes an "hospital exemption" applicable to the above described situation, a recent Europe-wide survey has shown significant variations in its application across European countries, thus generating significant disparities in patient access to these new form of therapies and clinical research ("Academic GMP" has produced its own contribution for the present consultation).
5) The hospital exemption also precludes exports of ATMPs produced within this regulatory frame: this may paradoxically favour the persistence of situations in which a "similar" rather than strictly identical or harmonized process will be used by different facilities located in different countries, rather than favour a centralized production. In addition, this is the experience of EBMT and other sister organizations that increased cross-border exchanges of allogeneic HSC grafts has contributed to increased harmonization in medical practices, cell collection procedures and cell engineering processes.
6) Publication of the regulation has not prevented the development of stem cell tourism that flourishes on the perception that needs for several severe ailments remain largely unmet.

EBMT is interested in working closely with European and national health authorities and competent authorities to further the development and use of ATMPs, particularly in its "historical" field in haematology and oncology. EBMT's members represent a reservoir of knowledgeable professionals that are familiar with the specificities of living cells and tissues as biological material intended for therapeutic use. EBMT believes that of the practical experience of its members, combined with that of representatives of industry and regulatory bodies can bring a pragmatic approach to accompany the development of ATMPs, and ultimately speed up progress in the field of HSCT and cell therapy in general.

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