

## **EBMT receives a regulatory qualification from the European Medicine Agency (EMA) on the use of its patient registry to support novel CAR T-cell therapies.**

**Barcelona, March 1<sup>st</sup> 2019** – The European Society for Blood and Marrow Transplantation (EBMT) registry has been qualified by EMA as a suitable platform for the collection of data for post-authorisation safety surveillance and efficacy studies. The registry is now considered suitable to perform pharmacoepidemiological studies for regulatory purposes, concerning Chimeric Antigen Receptor (CAR) T-cell therapy used in the treatment of haematological malignancies.

CAR T-cell immunotherapies are advanced therapy medicinal products (ATMPs) manufactured from human primary living cells procured by hospitals or blood banks. While clinical results of CART-cell products so far have been impressive, these highly personalised medicines can also have substantial adverse effects leading to severe complications in patients including death. Furthermore, some ATMPs may be considered as a once-in-a-lifetime treatment and long-term follow-up is needed to demonstrate the sustainability of efficacy. This is where registries such as EBMT become essential actors with a proven long-term follow-up of large cohorts of patients undergoing complex therapeutic procedures such as haematopoietic cell transplantation and immune effector cells.

The EBMT developed a specific Cellular and Gene Therapy Form to standardise registration of patients treated with cellular therapies such as CAR T-cells in Europe. Furthermore EBMT contributed to the EMA-facilitated consensus with regulators and industry on establishing the minimal dataset needed for long-term surveillance of patients receiving CAR T-cells.

This announcement comes as a result of extensive interaction with EMA starting in late 2016 when the EBMT first responded to the [EMA's Patient Registry Initiative](#) and subsequently in the formal qualification opinion request process.

Jürgen Kuball, EBMT treasurer and liaison with the EMA, says: "The EBMT is proud that EMA recognises the value of its registry. This recognition will lead to improved communication among the various stakeholders, including registry owners, regulators and marketing authorisation holders, giving confidence to users on the data collected and ultimately bring safe and effective therapies to our patients." He adds: "This has been a true collaborative effort with different stakeholders including additional volunteers from EBMT such as Chiara Bonini and Christian Chabannon, with the essential support of EBMT staff including Eoin McGrath, Carmen Ruiz de Elvira and Anja van Biezen."

For more information, please read the [full qualification opinion report](#)

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### **About the European society for Blood and Marrow Transplantation (EBMT)**

The European Society for Blood and Marrow Transplantation (EBMT) is a not-for-profit medical and scientific organisation established in 1974. It is dedicated to fighting life-threatening blood cancers and diseases and improving patients' lives.

The EBMT Members - more than 4,000 physicians, nurses, scientists and other healthcare professionals - participate in a unique collaborative network of peers involved in hematopoietic stem cell transplantation (HSCT) and cellular therapy research.

The EBMT patient Registry was established in the early 1970's. It is the only data source of its kind in Europe. The data are submitted continuously by more than 600 centres, including from outside Europe. The Registry holds data on more than 600,000 HSCT procedures including details on disease, transplant type, donor type and stem cell source. It contains data on patients that have undergone HSCT and also on patients receiving immunosuppressive therapies or other cell therapies. The Registry also offers the possibility to enter donors' follow-up data, which is crucial to ensure maximum donor safety. The Registry underpins extensive European research that translates into changes in clinical practice and improvements in patient outcome and care.

For further information about the EBMT, please visit the website: <https://www.ebmt.org> and follow us on Twitter: @TheEBMT

#### **Press contact**

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