EBMT
European Society
for Blood and Marrow Transplantation

CAR-T cells
Post Authorization studies

Background

Chimeric antigen receptor T cells (also known as CAR-T cells) are T cells that have been genetically engineered to recognize cancer cells in order to more effectively target and destroy them. Clinical results of CAR-T cell therapies have been impressive, however many questions regarding the personalized medicines remain, particularly concerning long term effectiveness and safety of the products.

The EBMT developed a specific Cellular and Gene Therapy Form to standardize registration of patients treated with cellular therapies such as CAR-T-cells. This module of the EBMT registry has been qualified by the European Medicines Agency (EMA) as a suitable platform for the collection of long term follow-up data for post-authorization safety and efficacy studies of CAR-T cell products.

Objectives

- Evaluate the long-term safety of patients treated with CAR-T Cells in a real world setting as measured by type and frequency of adverse events.
- Determine the overall survival rate and causes of death after administration of CAR-T Cells.
- Evaluate the efficacy and the timet on next treatment after administration of CAR-T Cells.
- Assess the safety and effectiveness profile by gender, age and in special populations.

Inclusion criteria

- Patients treated with commercial CAR-T products
- Patients who have given informed consent

Participation

Countries reporting CAR-T cell treated patients to the EBMT Registry

Source: EBMT Registry, January 2021

- The numbers of centers and countries registrering CAR-T Cell patients is growing

96 sites provide data now!

Thank you for registering CAR-T patients!!!

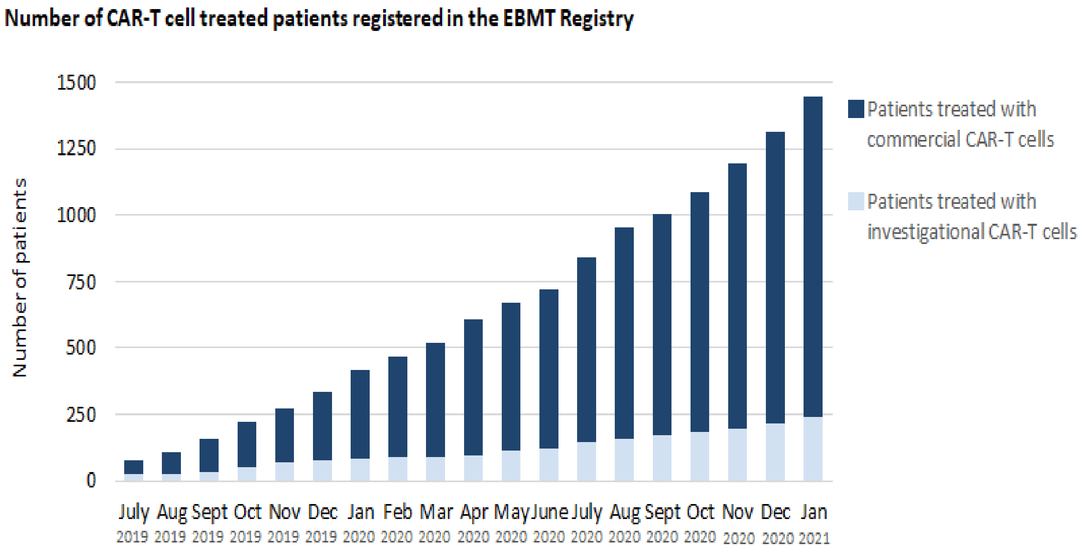
Regulatory requirements

- For proper evaluation of the safety profile of the CAR-T Cells, patient level data is needed.
- Patient level data can be shared with EMA and MAHs only if Informed Consent Form is signed off. For the purpose, the ICF needs to be approved by the respective regulatory organs.

Please prioritize regulatory approvals of the ICF in your countries to be able to consent patients

Data

Number of CAR-T cell treated patients registered in the EBMT Registry



Source: EBMT Registry, January 2021

Important points for data registration

Registration in Promise
- EBMT center members and national registries are requested to register CAR-T patients.

Batch number:
- Please enter the Company's product specific batch number.

Complication (infectious/non-infectious)
- Report in the Registry as per available information in patient files.

Please provide for each of the infectious and non-infectious complications, if they occurred or not.

Cytokine release syndrome (CRS) and neurotoxicity
- Please use ASTCT Consensus Grading scale.

Hypogammaglobulinemia
- Was hypogammaglobulinemia present before the cellular therapy?
- If Yes, was it worsened by the cellular therapy?

Secondary malignancy
Report all secondary malignancies.

• Follow up status
Indicate if patient is alive / death/ lost to follow up.

For the MAHs to fulfill their EMA obligations and provide complete and up-to-data safety CAR-T data, we need to obtain the patient data on an ongoing basis. Therefore, please provide regularly EBMT with all your CART patient data!

Contact

Please send your data to EBMT_NK01@lumc.nl

If you have questions, please contact your country lead or send your question to EBMT_NK01@lumc.nl

EBMT Clinical Study Unit

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