

### 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 these 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 time to 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 CAR T-cell products
- Patients who have given informed consent

### Participation

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



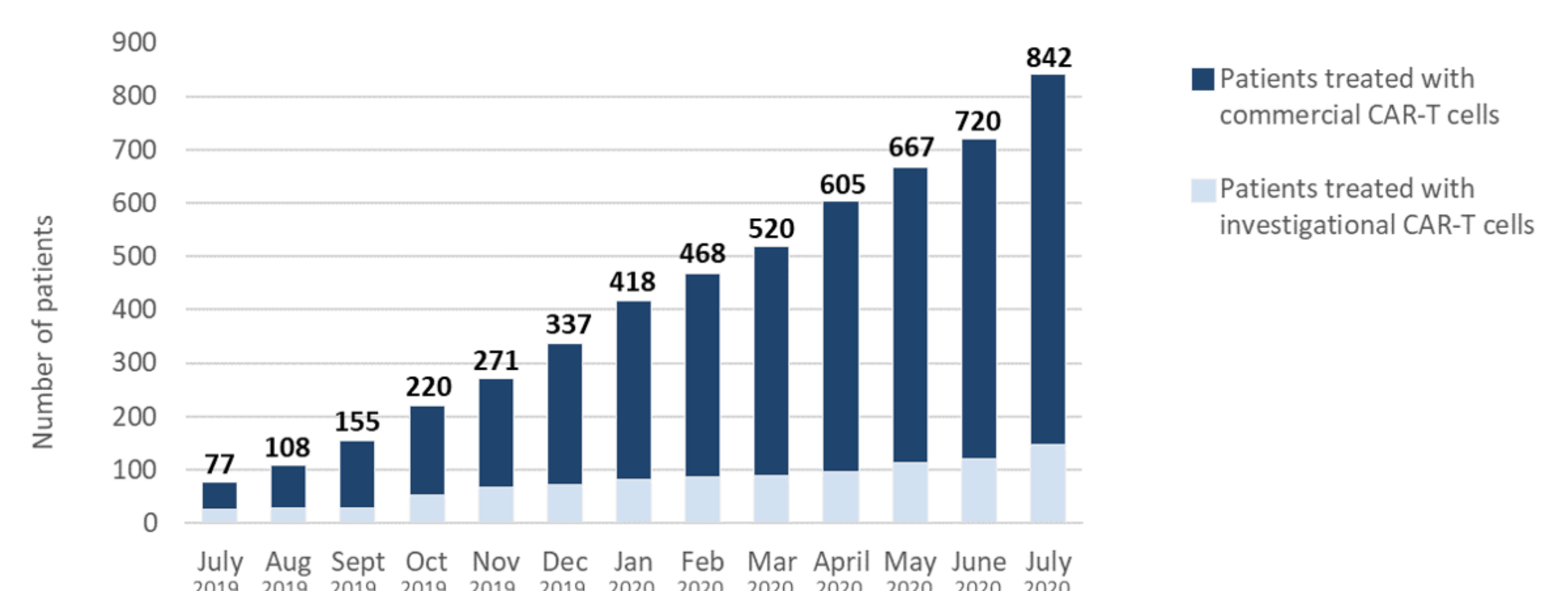
Source: EBMT Registry, July 2020

**The number of centers and countries registering CAR T-cells is growing! Thank you for registering the CAR-T cell data!!!**

- Please prioritize data registration of commercial over investigational CAR T-cell products.
  - EBMT is also interested in registration of patients treated with investigational CAR T-cells. The data of investigational CAR T-cell products will be used only if the investigator/sponsor provides permission for use.
- **‘GoCART’-initiative is underway!**  
The initiative aims to create a multi-stakeholder network of all interested parties to maximize the potential of CAR T, focusing on:
  - Pre- and post-marketing registry
  - CAR T-center accreditation
  - CAR T-cell educational program
  - CAR T-cell policies and legislation

### Data

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



Source: EBMT Registry, July 2020

### Data Forms

EBMT center members and national registries are requested to complete:

#### Cellular Therapy form

The form was designed to capture essential information describing the administered product and patient follow-up. The form can be found on our site <https://www.ebmt.org/registry/data-collection>

#### Data request

There are additional items that are not captured in ProMISE and that are collected in a separate data request using excel.

**Please send in your CAR-T cell data by completing the forms!**

### Important points for data registration

#### Complications (infectious/non-infectious)

- Provide each complication on the follow-up forms
- Use the ASTCT consensus grading for cytokines release syndrome and neurologic toxicity associated with Immune Effector Cells, as described in [Biol Blood Marrow Transplant, 2019 Apr;25\(4\):625-638. doi: 10.1016/j.bbmt.2018.12.758. Epub 2018 Dec 25.](#)

#### Commercial products

Cell infusion unit		Manipulation of the cell infusion unit			
Name of marketing authorization holder	Name of the (CIU) package/product	Gene transfer	Target	Suicide gene	Transgene TCR
Novartis	Tisagenlecleucel (Kymriah)	Yes, Lentoviral vector	CD19	No	No
Kite Gilead	Axicabtageneicel (Yescarta)	Yes, Retroviral vector	CD19	No	No

- Dose: use the specifications on the label of the commercial product

#### Previous therapy

- Please register any line of therapy given before the Cellular therapy

#### Unavailable data

- Complete “unavailable” or “unknown” if data is really not available

#### Batch number

- Enter Company’s specific batch number which is reported on the label of the product (this may be kept in the pharmacy)

#### Molecular markers:

- Provide molecular markers

### Contact

Please send your data to [CTIWP@lumc.nl](mailto:CTIWP@lumc.nl)

Questions? Contact [EBMTCTO@lumc.nl](mailto:EBMTCTO@lumc.nl)



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