Gene and Cell Therapies in Oncology

How can a collaborative CAR-T cell registry advance developlement?

Hybrid Workshop, November 30th, 2021

Nicolaus Kröger, EBMT President

@TheEBMT

EBMT is a not-for-profit medical and scientific organisation

We are dedicated to **saving the lives** of patients with blood cancers and other life-threatening diseases by advancing the fields of blood and marrow transplantation and cell therapy worldwide through **science, education and advocacy.**

Membership across the globe

We have more than 5,000 members within over 570 centres.

512 Full Reporting Centre Members

169 Individual Members

58 Associate (non-reporting) Centres

* Data reflects EBMT Membership in 2020 (Source: EBMT Annual Report 2020).

Science
The EBMT Registry

The Registry contains **patient clinical data**, including aspects of the diagnosis and disease, first-line treatments, **HSCT or cell-therapy-associated procedures**, transplant type, donor type, stem cell source, complications and outcome submitted continuously by EBMT centre members.

In order to retain Full EBMT Membership status, **EBMT centres must report** all consecutive hematopoietic stem cell transplants each year.

The EBMT Registry

- The Registry database has data that goes back to the early **1970's** and is the **single biggest data source of its kind in Europe.**
- The registry collects data in more than **> 500** centres and from more than **> 50** countries.
- It receives nowadays **> 30,000** new HSCT registrations per year.
- Currently contains data on more than **> 600.000** HSCT procedures.

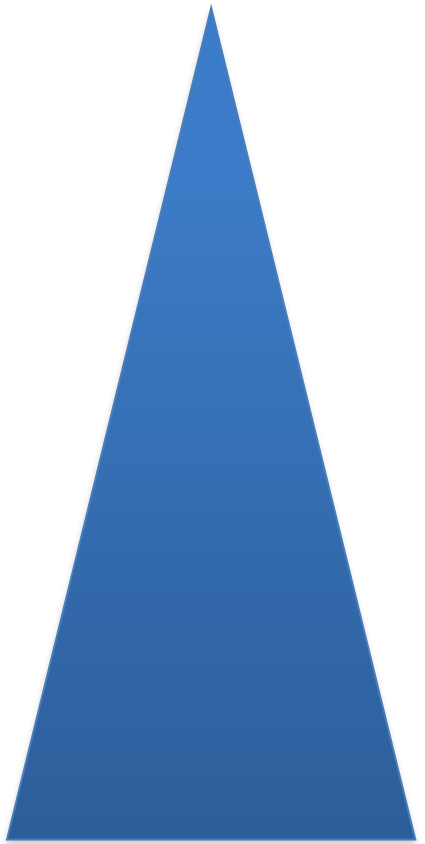
Algeria
Australia
Austria
Belarus
Belgium
Bulgaria
Canada
Colombia
Croatia
Czech Republic
Denmark
Estonia
Finland
France
Germany
Greece
Hungary
Iran
Ireland
Israel
Italy
Jordan
Latvia
Lebanon
Lithuania
Malasya
Netherlands, The
New Zealand
Nigeria
Norway
Poland
Portugal
Romania
Russia
Saudi Arabia
Serbia and Montenegro
Slovakia
Slovenia
South Africa
Spain
Sweden
Switzerland
Tunisia
Turkey
United Kingdom

Data ownership, consent, control, and use

- The OWNER is the PATIENT.
- The focus has to be on CONSENT, CONTROL and USE.
- Definitions taken from the General Data protection Regulation (GDPR) show EBMT as the CONTROLLER, and the LUMC in The Netherlands as the DATA PROCESSOR (because they host the database).
- EBMT as the CONTROLLER is currently taking all the necessary steps to comply with the GDPR in time for May 2018.

Data completeness and quality

1. Classical registry
2. Benchmarking (upcoming)
3. Retrospective EBMT studies
4. NIS
5. Clinical study



Science
EBMT Working Parties

The EBMT has **10 Working Parties** whose mission is to implement the EBMT's Scientific and Educational policies.

Together with the EBMT Clinical Research Department, they **develop and manage scientific proposals**. Plus, **all EBMT Registry studies** are performed under the supervision of the EBMT Working Parties.

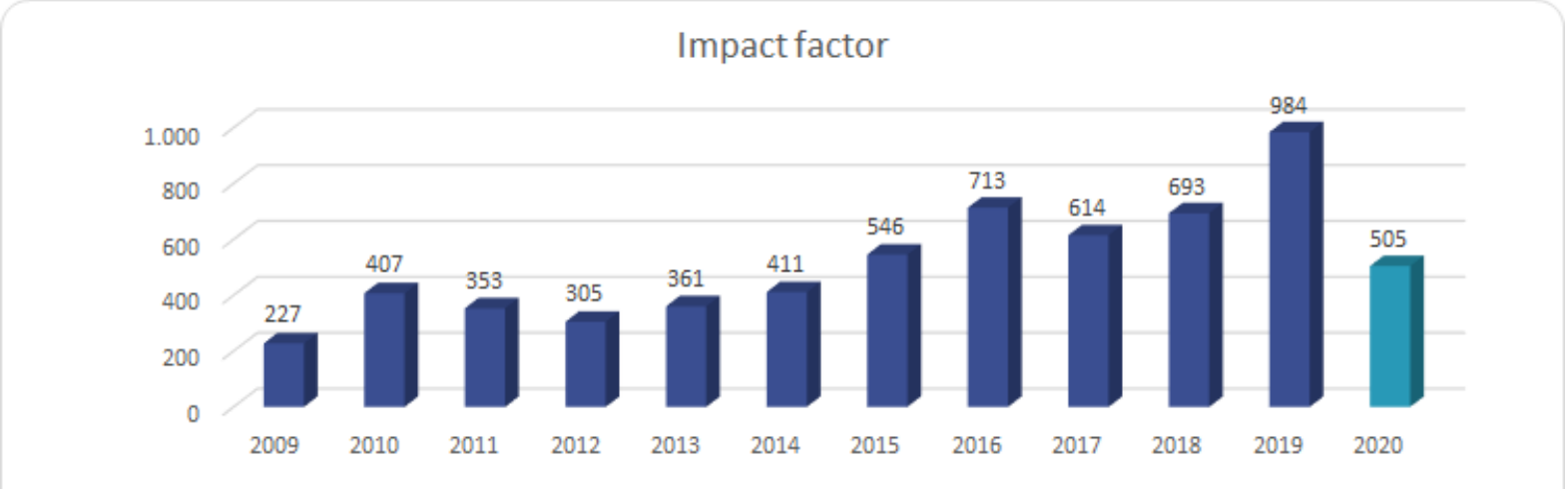
Science
EBMT Publications Studies

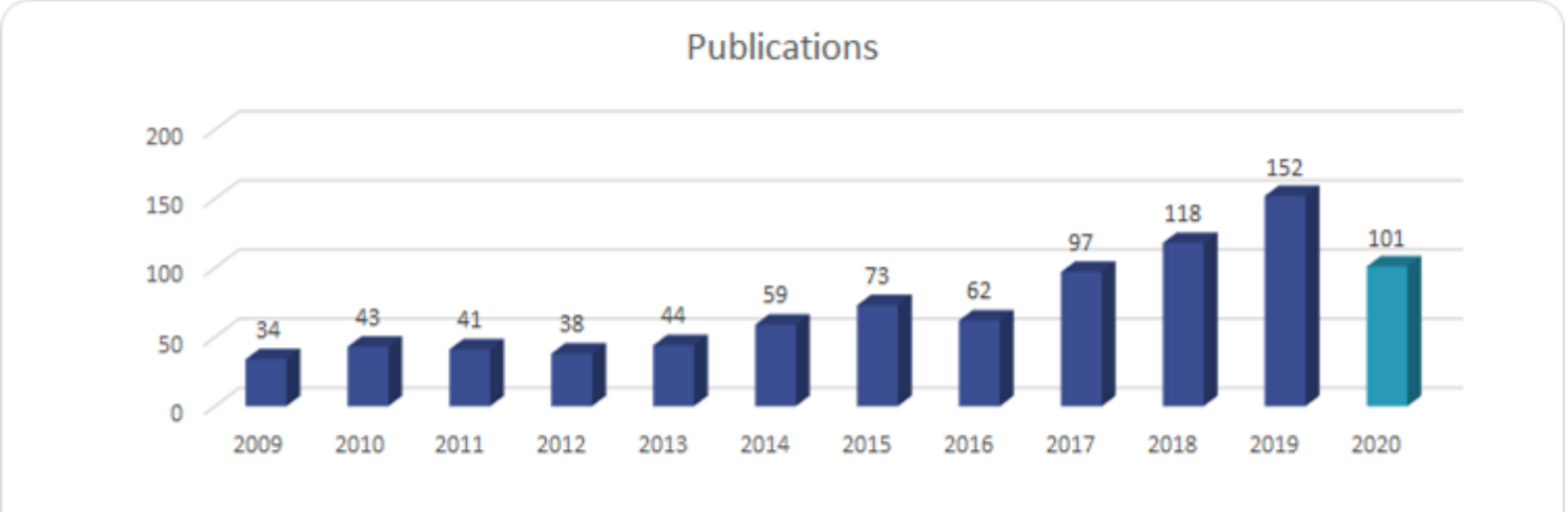
The data of the EBMT Registry is used to perform **four types of studies**:

1. Retrospective data collection studies
2. Non-interventional cohort studies
3. Interventional studies (Prospective clinical trials)
4. Post-Authorisation studies

EBMT publishes in top ranking **peer reviewed journals** year after year.

Science
EBMT Publications Studies





* Data Source: EBMT Annual Report 2020.

Benchmarking models

- Benchmarking is a new requirement for centers operating under JACIE accreditation
- Benchmarking is in contrast to the US not yet operational in all European countries
- EBMT has been taken its responsibility to develop a benchmarking model suitable for the European market
- This will create an additional incentive for complete and accurate reporting to the registry

Statistical models are developed in collaboration with LUMC, NL

Why should we register patients undergoing cell and gene therapy?

- **Capture “Real life” data**
- Collect even rare and long-term AE
- Compare clinical results of cell and gene therapy products with gold standard therapeutic approaches
- Increase collaborative interactions between Cell and Gene Therapy stakeholders

Limitations that registries can address (1)

Creating a strong and comprehensive data set

- **Sponsor-specific registries can create 'silos'** which blocks sharing and comparison.
- **A single reporting resource will avoid fragmentation** of small data sets
- **Strongly contribute to standardisation** by establishing and enforcing definitions across countries and diagnoses.
- This will allow to **achieve a meaningful body of pooled data,** which could be used to generate aggregate reports for third parties such as regulators

Limitations that registries can address (2)

Real world testing

- Testing whether benefits observed in clinical trials are also seen in unselected patient populations in **real-world settings**
- **Justify public investment** to ensure availability of tissue and cell therapies

Covering the lifetime of a patient

- A **single individual** patient can sequentially receive **several types and categories of cellular therapies in combination** with other categories of treatments and so a broader data capture strategy than just a therapy-specific focus is necessary.

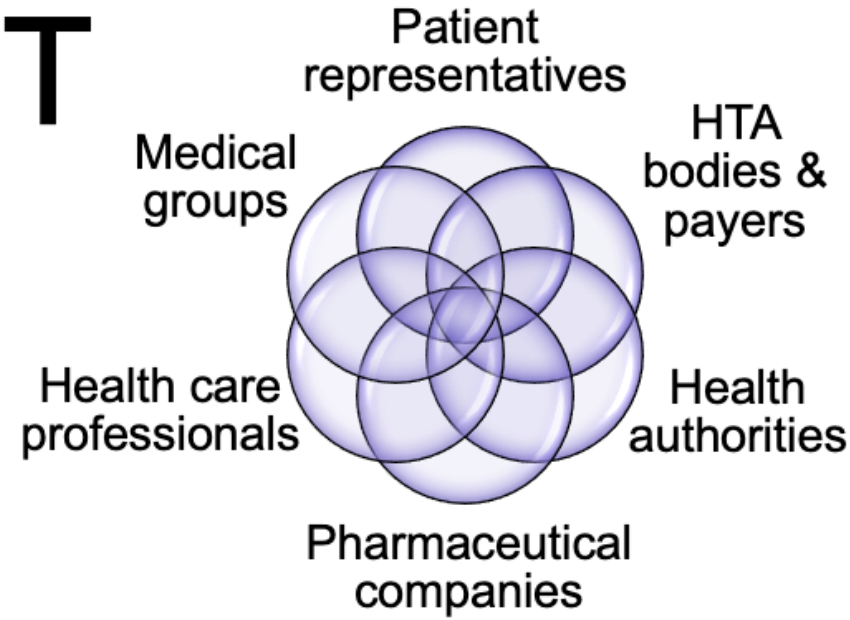
Multi-stakeholder Coalition in the field of gene and cellular therapies

GoCART COALITION

Vision: Be a trusted partner and a leading force in the field of gene and cellular therapies at national and international levels

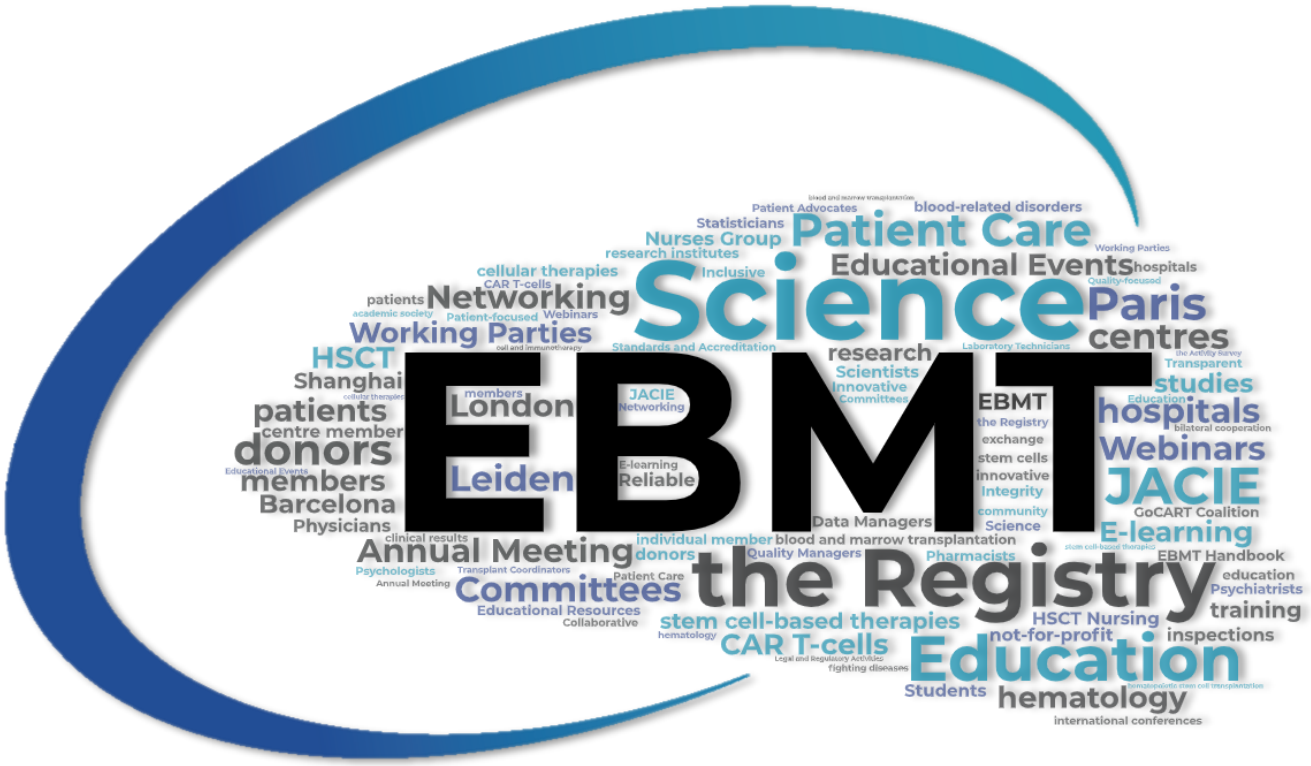
Mission: Promote patient access to novel gene and cellular therapies and to contribute health and well-being through multi-stakeholder collaboration on clinical data, standards of care, education and policy

Visit our website:
www.theGoCARTcoalition.com



Join our work package activities ▼

- WP1 Data harmonisation
- WP2 Standards of Care
- WP3 HTA
- WP4 Education
- WP5 Policy and Advocacy
- WP6 Scientific Excellence



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