EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Chimeric antigen receptor (CAR) T cell therapies

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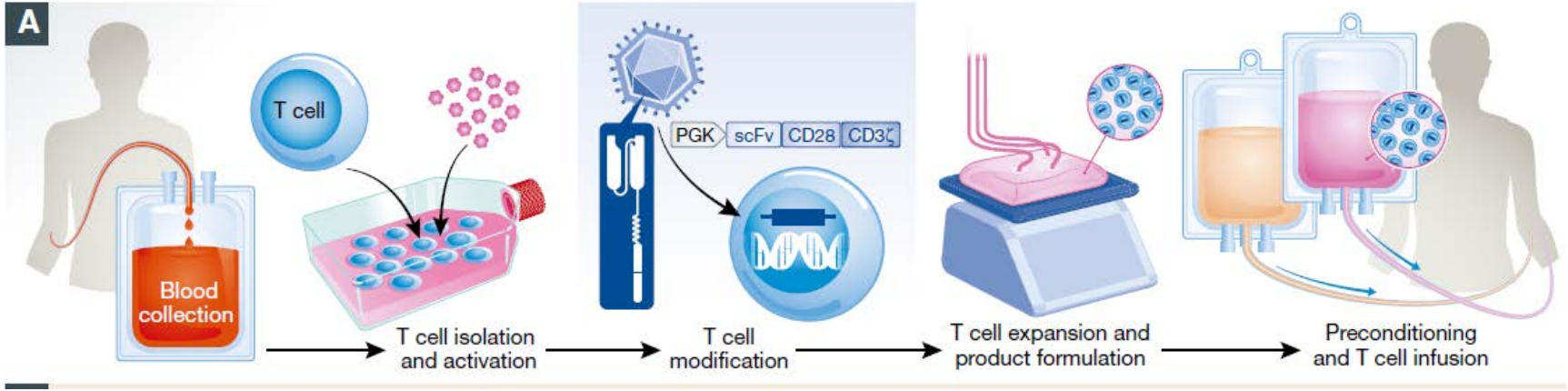
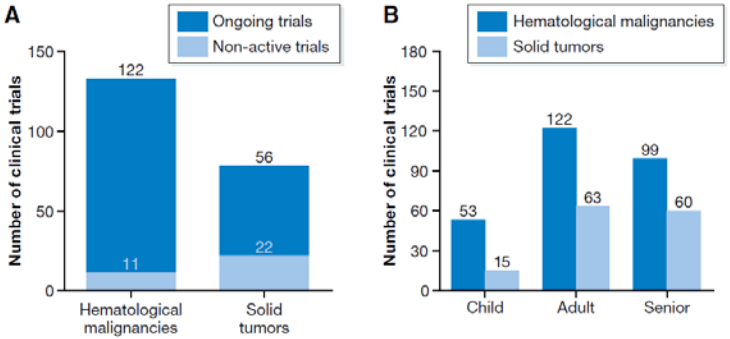
CAR T cells

- Next generation of immunotherapy for the treatment of cancer
- Generation of genetically-engineered T-lymphocytes (white blood cells) derived from the patient's own blood (autologous) or derived from a healthy person (allogenic)
- Cells genetically modified to express a receptor directed against surface components of tumour cells which directs them to the tumour to kill the cell
- Currently being developed primarily to treat leukaemia and lymphoma although it is likely that this portfolio will expand to treat solid tumours

CAR T cells





Hartmann et al (2017)
EMBO Mol Med (2017) 9: 1183–1197

CAR T cells

- Currently two products being considered by the CAT for marketing authorisation
- FDA has approved Yescarta (axicabtagene ciloleucel) to treat adult patients with certain types of large B-cell lymphoma
- Challenges
  - Some deaths in early stage trials (Juno Therapeutics) due to cerebral oedema
  - Toxicity can be associated with increase in cell number following transfusion
  - Variability in results between patients in individual studies
  - Differential dose requirements based on individual patient-derived cells and inter-subject variability
- EMA CAR T cell workshop in Nov 2016 with industry and academic researchers

The use of registries for CAR T cells

- Potential to use existing registries to follow patients following treatments and compare the different therapies, especially considering potential toxicities
- European Bone Marrow Transplant (EBMT) registry currently under consideration
- EMA organised a meeting to discuss the potential use of registries with multiple stakeholders

Agenda – CAR-T cell therapy Registries Workshop

9 February 2018, 08:30 to 16:30 UK time

- To facilitate the long-term follow up of CAR-T cell products in a real world setting and enable the generation of meaningful efficacy and safety data using haemato-oncological registries
- To agree on implementable recommendations on core data elements to be collected, patient consent, governance, quality assurance and registry interoperability.
- To agree on recommendations to optimise collaboration among registry holders, MAHs/MAAs and regulators