EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

CAT update September 2018

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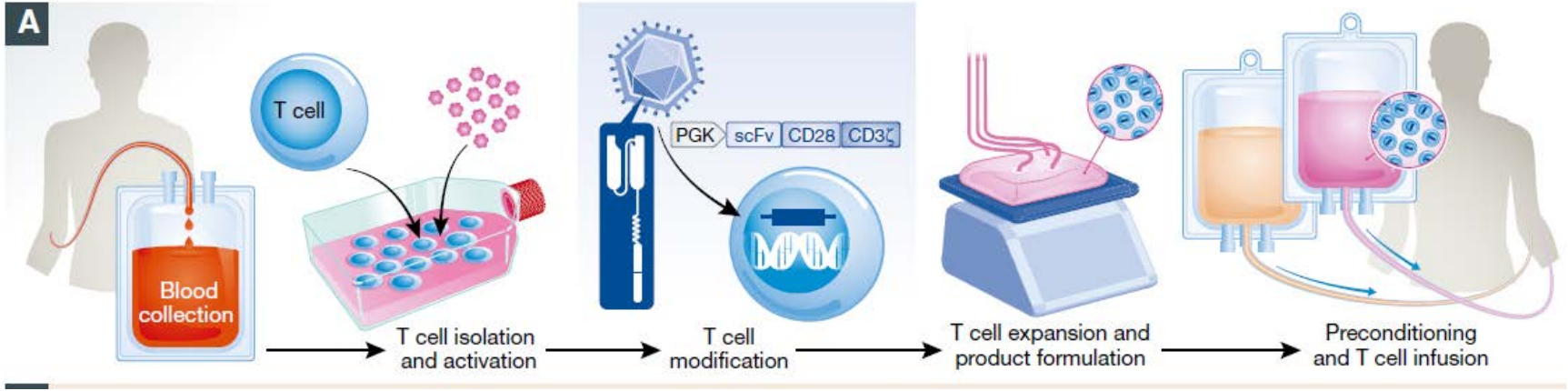
Committee for Advanced Therapies

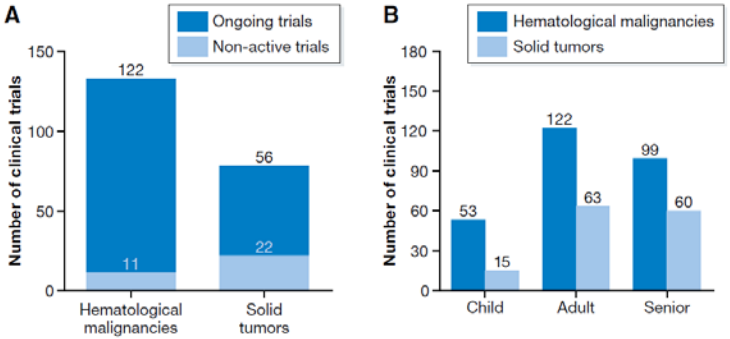
An agency of the European Union

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

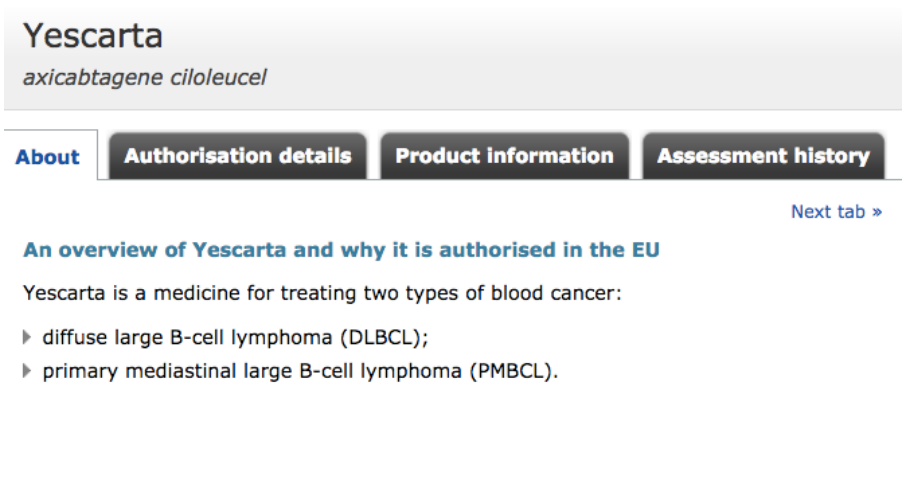


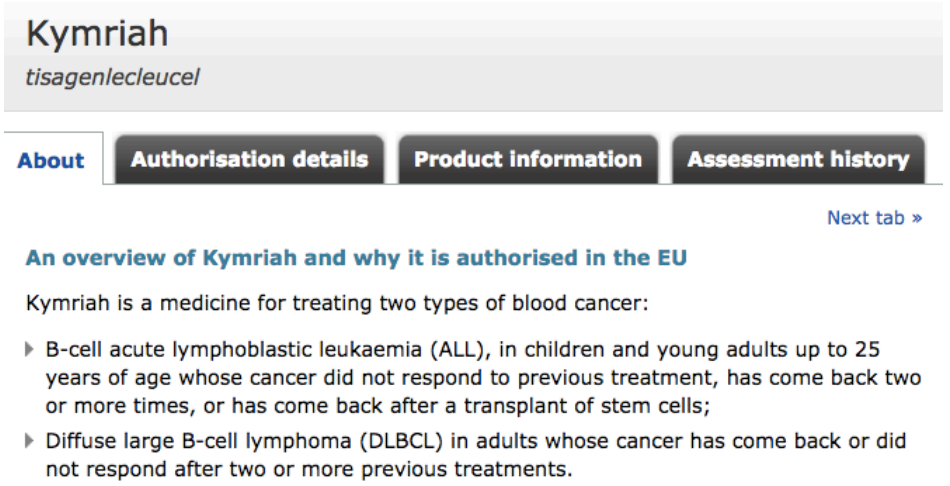


Hartmann et al (2017) EMBO Mol Med 9: 1183–1197

CAR T cells

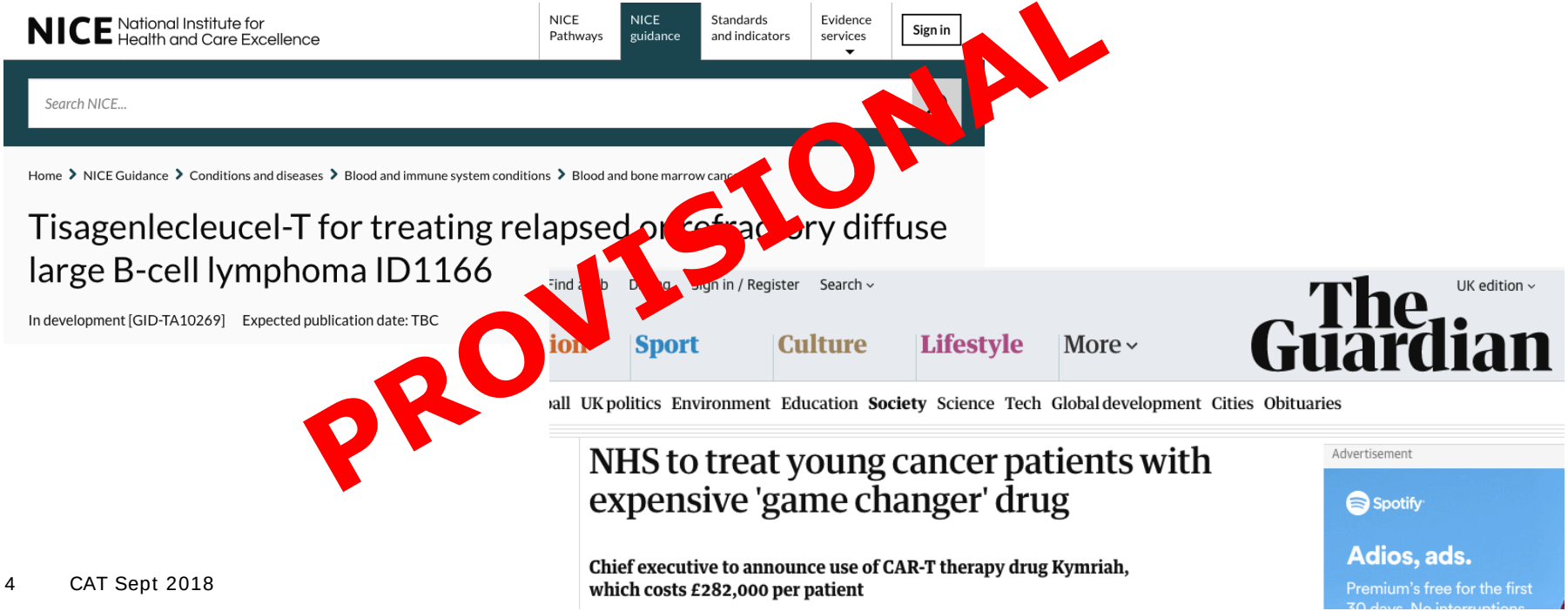
- Two products approved by European Medicines Agency and European Commission (Aug 2018)





CAR T cell approval for reimbursement by UK HTA (NICE)

Kymriah



CAR T cell approval for reimbursement by UK HTA (NICE)

Kymriah

PharmaTimes online

Search news, artic

NICE rejects Novartis' CAR-T Kymriah for adult lymphoma

NICE National Institute for Health and Care Excellence

NICE Pathways | NICE guidance | St ar

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NICE encourages further discussions on Kymriah for adult lymphoma

CAR-T is too expensive to recommend as a treatment for adults with lymphoma, NICE says in draft guidance today (19 September).

CAR T cell approval for reimbursement by UK HTA (NICE)

Yescarta

NICE National Institute for Health and Care Excellence

NICE Pathways | NICE guidance | Sta anc

28 August 2018

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Feedback encouraged to allow use of life extending treatment on NHS for those with blood cancer

CAR-T considered to be an exciting innovation which can cure some patients but very expensive

Mail Online

Children with leukaemia and adults with lymphoma face being DENIED 'cancer cure' as NHS officials and pharmaceutical bosses argue over pricing

The use of registries for CAR T cells

- European Bone Marrow Transplant (EBMT) registry currently under consideration to follow patients following CAR T cell treatments and compare the different therapies, especially considering potential toxicities
- Initial meeting in Feb 2018 to discuss the potential use of registries with multiple stakeholder resulting in a qualification recommendation (out to consultation)

Draft qualification opinion on Cellular therapy module of the European Society for Blood & Marrow Transplantation (EBMT) Registry

Agreed by Scientific Advice Working Party	17 May 2018
Adopted by CHMP for release for consultation	31 May 2018*
Start of public consultation	29 June 2018†
End of consultation (deadline for comments)	21 August 2018‡

CAT Workplan 2018

- Revision of the guideline on genetically modified cells
- Development of a guideline on requirements for ATMPs in clinical trials
- Development of guidance on comparability for ATMPs
- Reflection on the use of Registry data for the post-authorisation follow-up of ATMPs.
- Scientific and Regulatory considerations on gene editing technologies
- Addressing the Environmental Risk assessment of ATMPs containing genetically modified organisms (GMO) / genetically modified micro-organisms (GMM).

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Questions?