Advanced Therapy Medicinal Products and NICE

Deborah Morrison - Senior Technical Advisor, NICE Scientific Advice
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NICE – aims:

- Speed up NHS uptake of interventions that are both clinically and cost-effective
  - Produce evidence-based guidance
- Encourage better and more rational use of resources by focussing the provision of healthcare on the most cost-effective interventions
  - Develop quality standards and performance metrics
- Encourage more equitable access to healthcare (reduce post code lottery of care)
  - Provide a range of information services
- Encourage the creation of new and innovative technologies
Changes to NICE Technology Appraisals and Highly Specialised Technologies Programmes

• Important changes were made in 2017 to support financial sustainability

• Budget Impact Test
  o All technologies are subject to a budget impact test
  o Technologies with a budget impact of more than £20m pa in the first 3 years of implementation may be subject to commercial negotiations with NHS England in addition to NICE cost effectiveness analyses

• Cost per QALY threshold introduced in the HST Programme
  o Previously took account of cost per QALY but there was no threshold against which to judge value
  o Thresholds of £100,000 - £300,000 per QALY introduced (applicable threshold depends on the magnitude of individual patient benefit)
NICE Regenerative Medicine Study

- Hypothetical Car-T Cell product

- Study allowed detailed consideration of specific challenges associated with the evaluation of ATMPs including the interplay between evidence maturity, price and payment methods

- Two reports:
  - Detailed technical report produced by the York team
  - Short overview report produced by NICE

https://www.nice.org.uk/about/what-we-do/science-policy-research/nice-research
What about the evidence?

- **RCTs**… rare… trials are short… sample sizes are small
- **Limited generalisability & external validity**
- **New trial designs**
- **Surrogate outcomes**
  
  *Future – outcomes? Unexpected adverse effects? Repeat administration?*

**Uncertainty**
How can the economic risk for ATMPs be reduced?

Two main approaches:

• Cost sharing arrangements:

Or

• Managed Access Agreements (Patient Access Schemes, Managed Entry Schemes or Performance Based Risk Sharing arrangements (PBRSA))

Can be linked to Health Technology Assessment (HTA)
The picture so far…

- Chondroselect - Autologous chondrocyte implant – **NO**

- MACI - full thickness cartilage defects of the knee –**NO**

- Autologous pancreatic islet cell transplantation for improved glycaemic control after pancreatectomy (IPG274)

- Provenge (Sipuleucil-T) - metastatic (non-visceral) prostate cancer – **NO**

- Talimogene laherparepvec (TA410) for treating unresectable metastatic melanoma – **YES**

- Holoclar (TA467)– ex-vivo autologous corneal epithelial cells containing stem cells – **YES (optimised)**

- Strimvelis (HST) – combined immunodeficiency caused by adenosine deaminase deficiency – **going through evaluation consultation now – YES?**
…..and what is coming next

• Alipogene tiparvovec (Glybera) – familial lipoprotein lipase deficiency – HST (block scoping report)

• Chondroselect, MACI and traditional autologous cultured chondrocytes (currently under hospital exemption) – technology appraisal

• Axicabtagene ciloleucel (KTE – C19, CAR-T) for treating relapsed or refractory diffuse large B-cell non-Hodgkin lymphoma – guidance in development

• KTE-C19 for treating relapsed or refractory mantle cell lymphoma - Topic selection

• Tisagenlecleucel-T (Kymriah (CTL019)), for children and young adults with B-cell ALL that is refractory or has relapsed at least twice

• Zalmoxis – genetically modified T cells – immunogene therapy – conditional marketing authorisation
Get in touch…

- www.nice.org.uk/scientific_advice
- scientificadvice@nice.org.uk
- www.nice.org.uk/OMA
- OMA@nice.org.uk

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