# How do we attain sustainability, affordability and availability of CAR T therapies?

**PANELISTS**

- Anne Kerber (BMS)
- Konstantinos Lykopoulos (Kite / Gilead)
- Tarang Sharma (World Health Organization Regional Office for Europe)

**MODERATOR**

- Amanda Whittal

## Topic Overview

**CAR Ts are fundamentally different from conventional therapies**

- High value, potential lifelong benefits
- Exceptionally complex manufacturing and delivery

**There are specific implications of these differences**

- These translate into largely economic challenges:
  - Continuous investment in manufacturing and delivery
  - Pricing & reimbursement (P&R) challenges
  - Long-term impact and affordability uncertainty

**Policy actions could support access to these life-changing treatments**

- P&R approaches that capture CAR T specificities
- Agreed approaches to manage long-term impact affordability and uncertainty
- Multi-stakeholder discussions to align on ways forward

## Perspectives on CAR T challenges and implications

### Clinical Development perspective

#### Challenges

- CAR Ts are complex to develop and deliver, requiring specific supply chain efficiencies, and specialised expertise and equipment to conduct real time processes under strict quality controls
- Economies of scale are not yet possible due to these complexities
- Trial design challenges are present due to lack of clinical equipoise in light of the transformative benefit of cell therapy products, which often need to use single arm trials or cross-over designs for ethical purposes
- Despite the magnitude of potential benefit to patients, challenges still exist around access and sustainability

#### Implications

- The current policy environment is not tailored to the complexity of medicines like CAR Ts, which significantly disincentivises their development
- Evolving legislation and restrictions on incentives could mean further challenges to access

### Market access perspective

#### Challenges

- The one-off payment for CARTs faces increased payer challenges vs treatments with longer administration schema, despite often being highly cost-effective due to potential long-term effects
- The complexities of CAR T development translate into costly production and commercialisation, compounded by challenging P&R negotiations in European countries
- Increasing cost containment measures, price erosion of even high-value indications and decreased willingness to engage in novel payment models are all increasing the risk of making the complex, high-cost development of CAR Ts unsustainable for manufacturers

#### Implications

- CAR Ts need adequate funding for sustained innovation, but also to enable hospitals to care for patients receiving these therapies
- Even when treatments are available, only about 1/3 of eligible patients are actually receiving treatment
- Current pricing and funding challenges create a real risk that companies could rethink the viability of future launches in Europe; we are at a stage in development where investment is needed to maintain innovation and build efficiencies

### Policy perspective

#### Challenges

- Uncertainty in evidence at conditional marketing authorisation and increasing unaffordability for payers/ health technology assessment bodies is a real issue
- Lessons learned from the The Oslo Medicines Initiative have led to prioritized technical areas
- Lack of trust, collaboration and alignment across countries and different stakeholders needs to be addressed

#### Implications

- Policymakers recognise that change needs to happen faster; they are working to address challenges on the policy side:
  - Priority areas and policy recommendations are being taken forward through the World Health Organization Europe's Novel Medicines Platform, which includes novel treatments such as CAR-Ts
  - Different stakeholders, including policymakers, are collaborating across four working groups in the space of "conditional reimbursement" to facilitate better alignment and early engagement
  - These working groups will develop concrete demonstration projects as proof of concepts

**CAR Ts are novel therapies and thus require novel, collaborative approaches to ensuring sustainability, affordability and availability**