Regulatory challenges for the next wave of cancer therapies

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Disclaimer and Conflicts of Interest

The opinions expressed are my own and not necessarily the opinions of the Janssen Pharmaceutical Companies of Johnson & Johnson, or any other organization or individual.

I am an employee of Johnson & Johnson, and a shareholder of Johnson & Johnson.

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About RSNN

- The Regulatory Science Network Netherlands (RSNN) is a **network of experts from industry, academia, government bodies**, and the broader regulatory science field.

- Our mission is to advance **an efficient and effective regulatory system** that supports medicines development, marketing authorization, access, and appropriate use of medicines.

- RSNN shares and **disseminates knowledge** among all stakeholders and **sets the agenda for further research.**

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Regulatory challenges for the next wave of cancer therapies

- A wave of **innovative oncology treatments** is progressing from bench to clinic.
- This includes treatment strategies such as chimeric T cell receptors (CAR-T) and other approaches that are categorized as **Advanced Therapy Medicinal Products** by the EMA.
- These products have the potential to transform the future of oncological treatment, **but require innovative approaches concerning the risk-benefit assessment and market access.**
- **Is the current regulatory landscape ready** to facilitate registration of these innovative therapies to patients at the earliest appropriate time?
- Can we learn from the hurdles and barriers in the current ATMP regulation to **prepare ourselves for new cancer therapies such as CAR-T?**

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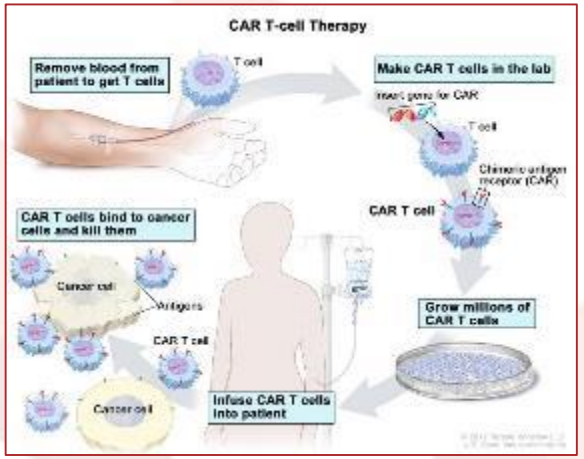
CAR-T Cell Therapy as an example of an ATMP that require innovative regulatory approaches

CAR-T cell therapy: How does it work?



Manufacturing CAR-T cells

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Today, 2 CAR-T products have been approved in US and EU

KYMRIAH® (tisagenlecleucel) Suspension for IV infusion

NOW APPROVED

YESCARTA™ (axicabtagene ciloleucel) Suspension for IV infusion

NOVARTIS

Kymriah® (tisagenlecleucel), first-in-class CAR-T therapy from Novartis, receives second FDA approval to treat appropriate r/r patients with large B-cell lymphoma

MAY 1, 2018

FOR MEDIA USE ONLY

- Kymriah demonstrated an overall response rate of 50%, with median duration of response not yet reached at the time of data cut-off, indicating sustainability of response[1]
- Kymriah is the only CAR-T therapy FDA-approved for two distinct indications – in non-Hodgkin lymphoma (NHL) and B-cell acute lymphoblastic leukemia (ALL)
- Novartis has established leadership based on first-to-launch CAR-T therapy experience, existing treatment center network and payor environment understanding, which helps to support access and anticipated patient demand
- Novartis continues to collaborate with the Centers for Medicare and Medicaid Services (CMS) on various value-based pricing initiatives

Please download/allow Adobe Flash Player to view this content

Kymriah, the First Gene Therapy, Arrives With a $475,000 Price Tag

NICE Hesitates on Kymriah, Wants Further Discussions

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

First two CAR-T cell medicines recommended for approval in the European Union

Press release

29/06/2018

First two CAR-T cell medicines recommended for approval in the European Union

Development of Kymriah and Yescarta supported through PRIME

The European Medicines Agency (EMA) has recommended the first two marketing authorisations for chimeric antigen receptors (CAR) T-cells medicines in the European Union (EU). Kymriah (tisagenlecleucel) and Yescarta (axicabtagene ciloleucel) are advanced therapies for blood cancer. They belong to a new generation of personalised cancer immunotherapies that are based on collecting and modifying patients' own immune cells to treat their cancer.

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Regulatory and Access Considerations

- **Development**
  - Issues of ensuring chain of custody (COC)and chain of identity (COI) vs considerations of patient confidentiality (GDPR)
  - Disconnect between academia, (start-up) companies and health agencies
  - GMO as well as CTA approval to conduct clinical studies required
- **Quality and manufacturing**
  - Practicalities of QP release, as each patients material is "a batch"
- **Regulatory review**
  - CAR-T should qualify as an ATMP in the EU
  - Committee for Advanced Therapy (CAT) acts as the primary coordinating body at EMA (not CHMP)
  - Suitability for PRIME procedure
  - Conditional approval and appropriate means of conversion
- **Commercialization**
  - Challenging Re-imbursement and Market Access
- **And so on……**

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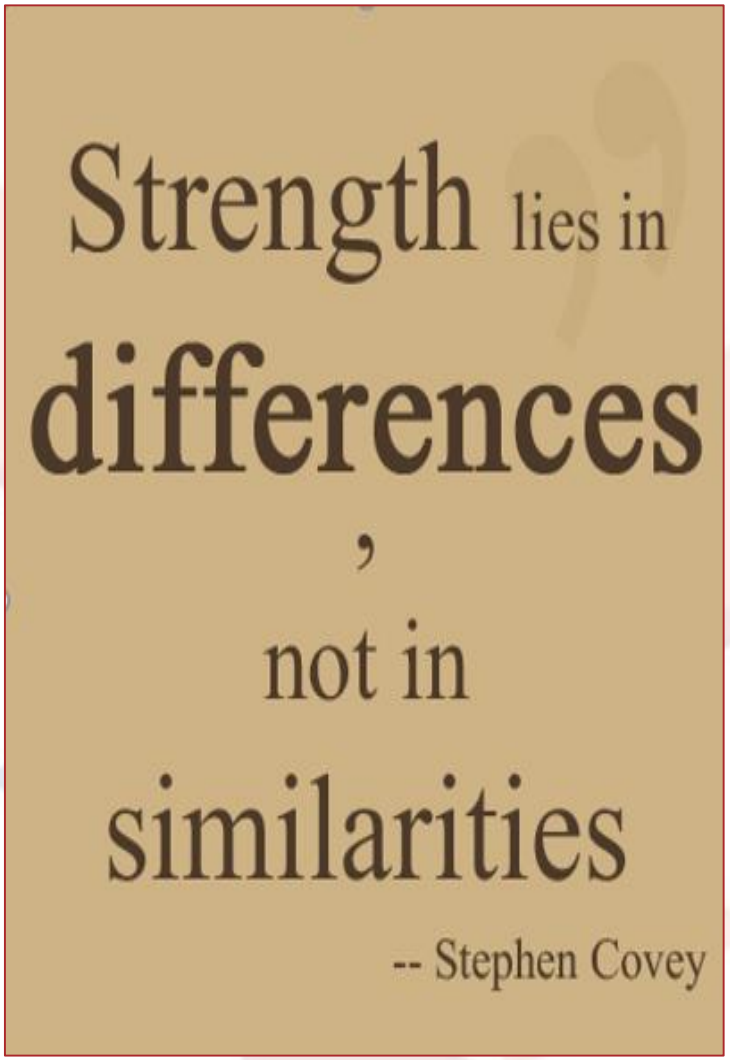

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Agenda

- Invited lectures:
  - **Regulatory considerations for ATMP based cancer therapies**
  - *Dr. Marcel Hoefnagel – Medicines Evaluation Board (CBG-MEB)*

  - **Planning for successful and efficient ATMP development**
  - *Dr. Harm Hermsen – Xendo*

  - **Access to innovative cancer therapies: the patient view**
  - *Dr. Pauline Evers – nfk*
- Selected abstracts:
  - **Challenges in EU Commercial ATMP Development**
  - *Renske ten Ham - Utrecht University*
- Panel discussion
- 11.00: closure

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