GeneTrack

Capture the Manufacturing Process, Perform Live Tracking, and Identify Individual Adverse Events of CAR-T Cell Therapy

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Presentation Overview

- Background: CAR-T Therapy
- Current Gaps
- Proposal: GeneTrack and GeneTracking Number
- GeneTrack System Demonstration
- Feasibility and Future Direction
- Conclusion
Background

- Chimeric antigen receptor-modified T cells (CAR-T) therapy
  - Most promising immuno-oncology therapy
    - High response rates in patients with R/R B-cell malignancies
  - Individuals CAR-T cell therapy are not the same
    - Quality in processing of gene reflects the safety and efficacy

Source: Image from Microbe.tv
How CAR-T Cell Therapy Works

1. Leukapheresis
2. Enrichment & Activation
3. Transduction
4. Expansion
5. Formulation & Quality Assessment
6. Administration

Source: Novartis Pharmaceuticals Corporation
Clinical Trials Landscape

- As of 2017, **only 2 FDA-approved** CAR-T cell therapies
- > 100 CAR-T cell therapy sponsored trials are being investigated in hematologic and solid tumor space

**Source:** Clinicaltrials.gov
Current Gaps

- **Limited tools** to capture the manufacturing processes of post-marketing CAR-T cell therapy and track its distribution
  - Little Transparency
  - Lack Universal Tracking System

- **Limited system** to report adverse events to individual CAR-T cell therapy
- **Uncertainties** in its long term safety and efficacy

Objective 3.4

- Foster improved manufacturing technologies and product characterization techniques through a combination of research and interactions with stakeholders including sponsors.

Importance of CAR T-Cell Regulation

• Novel individualized therapy
  – Required compliance of the CAR-T cell manufacturing process with global regulatory requirements
  – Limited long term data on Safety and Efficacy

• Expensive process

• Cumbersome/multiple manufacturing steps
  – Challenges in regulatory convergence or harmonization among the global regulatory agency
Proposal: GeneTrack System

- Publicly accessible database in compliance with HIPAA
- Capture the manufacturing process of the individual CAR-T cell therapy
- Report adverse events per individual CAR-T therapy
- Consolidate patient electronic health record (EHR) with GeneTracking number
- GeneTracking number will be linked to RFID/NFC technology
  - Live tracking of distribution
  - Tampered/Damaged detection
  - Storage temperature detection
Proposal: GeneTrack System
GeneTrack System Demonstration

GeneTrack is an extensive publicly accessible database that contains information on the collection, extraction, and manufacturing of individualized gene therapy. Along with the GeneTracking number, this database creates a transparent system to capture the manufacturing process and to perform live tracking of the individual gene therapy.

**Step 1:** To generate a GeneTracking number, please click [here](#).

**Step 2:** To fill relevant information regarding the collection, extraction, and manufacturing of individual gene therapy, please click [here](#). (Note: you must obtain a GeneTracking number to proceed)

To access the GeneTrack Database for manufacturing process information and live tracking of the individual gene therapy, please click [here](#). (Note: you must obtain a GeneTracking number to proceed)

**Unique GeneTracking Number**

i.e. a06c1107b1f8392b13d

**Vaccines, Blood & Biologics**

Home > Vaccines, Blood & Biologics > Cellular & Gene Therapy Products > GeneTrack

Source: Images/Figures have modified from the original FDA.gov Website
GeneTrack Online Form (step 2)

GeneTrack Online Form
Welcome to GeneTrack Database

Begin as a:

1. Name of therapy
2. Indication
3. Previous Treatments
4. Facility Location
   a) Hospital/Institution
   b) Manufacturing Center
5. Leukapheresis procedure
   a) Cell source
   b) Buffer condition
   c) Centrifuge rate
   d) Washing condition
6. Additional consideration

1. Components and Materials
   a. Vector i.e. component
   b. Reagents source
   c. Excipients

2. Procedure (i.e. Modification)
   a. Facility involvement
   b. Preparation
   c. Processing
   d. Final Formulation

3. Product testing
   a. Microbiology testing
   b. Identity testing
   c. Purity/potency testing

4. Additional consideration

Source: Images/ Figures have modified from the original FDA.gov Website
Hospital/Institution Form

GeneTrack

GeneTracking Number: *

a06c1107b1f83f2b13d

Name of Therapy (including Brand and Generic)

Kymriah (tisagenlecleucel)

Indication

Relapsed/Refractory B-cell Acute Lymphoblastic Leukemia

Previous Therapies Used for this Indication

Pegasparagase, vincristine, dexamethasone

Source: Images/ Figures have modified from the original FDA.gov Website
GeneTrack Database

GeneTracking Number: a06c1107b1f83f2b13d
Name of Therapy: Kymriah (tisagenlecleucel)
Type of Gene Therapy: Autologous

Live Tracking: Saturday, December 30
Shipment departed from Gene BioScience facility

VIEW ALL SECTIONS | CLOSE ALL SECTIONS

- INDICATION & PREVIOUS THERAPY
  Indication: Relapsed/Refractory B-cell Acute Lymphoblastic Leukemia
  Previous Therapy: Pegaspargase, vincristine, dexamethasone

- COMPONENTS & MATERIALS
  Cell source: Immune T-cell

- FACILITY OF PROCESSING
  Hospital/Institution: Gene Institution/Hospital
  Manufacturing Center: Gene BioScience

Source:
1. Images/Figures have modified from the original FDA.gov and NIH.gov Websites (AccessGUDID)
MedWatch Integration

Source: Images/Figures have modified from the original FDA.gov Website
Feasibility and Future Direction

• Feasibility
  – Easy implementation with already existing database framework (i.e. Global Unique Device Identification Database (GUDID))
  – Minimal cost to implement GeneTrack compared to the drug cost per treatment

• Future Direction
  – Incorporate real-world evidence across the total product lifecycle to improve patient safety and health outcomes.
Conclusion

● Limited tools available to capture CAR-T manufacturing and post-market distribution

● GeneTrack has many advantages
  ○ Improve transparency
  ○ Streamline the manufacturer process
  ○ Enhance the communication between multi-stakeholder (Patients, Stakeholders, and Industry Sponsors)
  ○ Individualize CAR-T cell therapy adverse events report
  ○ Easy implementation
Acknowledge

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Thank you for your time! Questions?