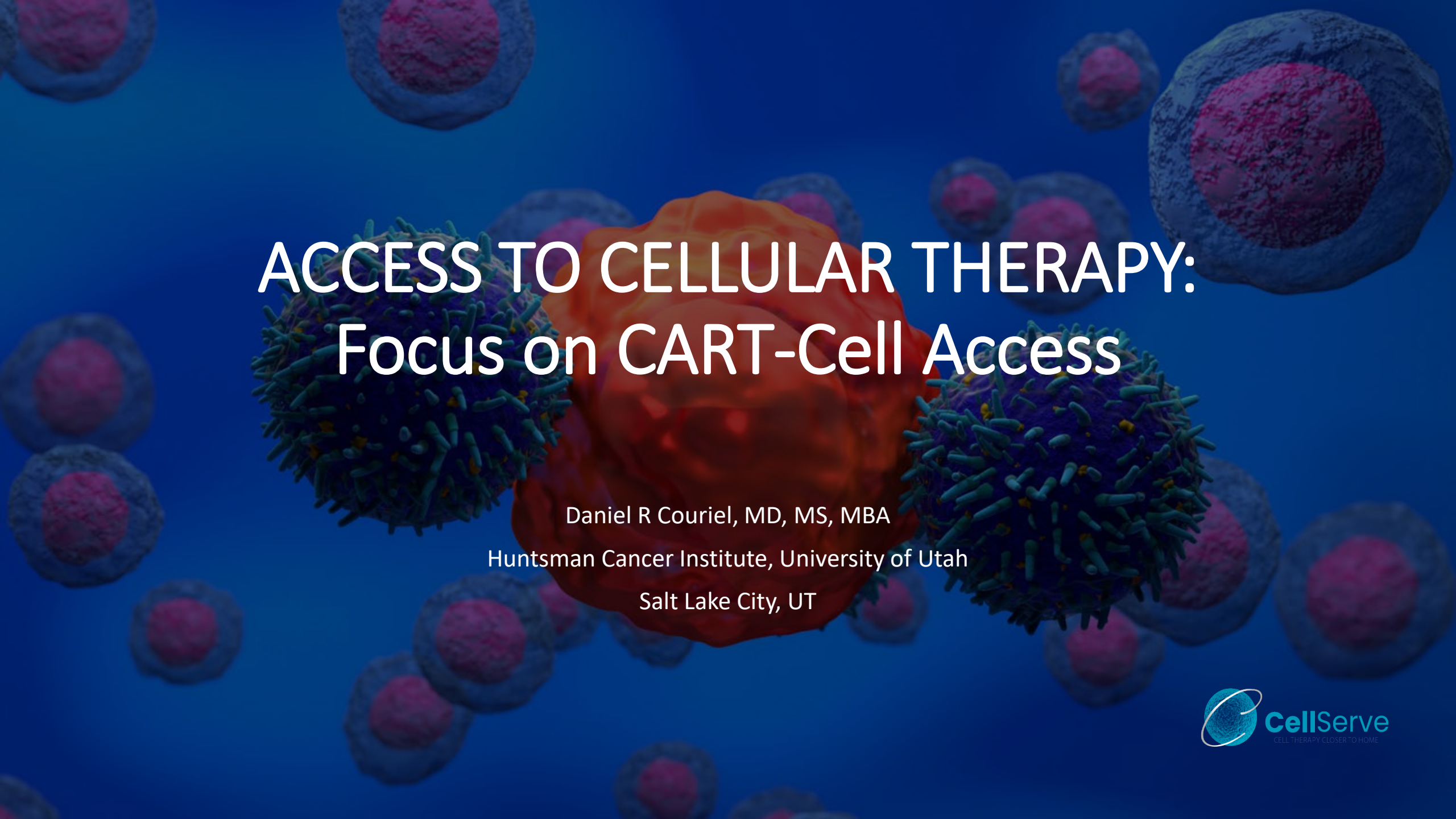


# ACCESS TO CELLULAR THERAPY: Focus on CART-Cell Access

Daniel R Couriel, MD, MS, MBA

Huntsman Cancer Institute, University of Utah

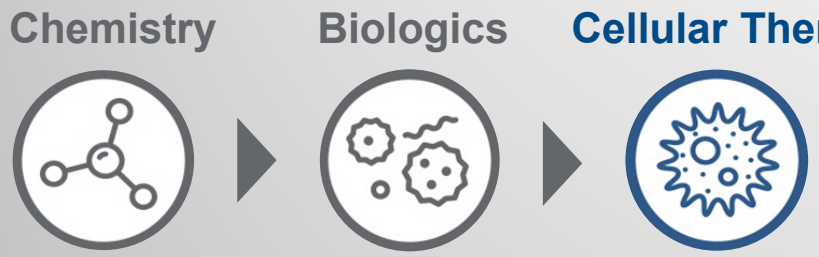
Salt Lake City, UT

CellServe

CELL THERAPY CLOSER TO HOME

# Cell and Gene Therapies: A True Revolution

**CAR T is a breakthrough Cellular Therapy**



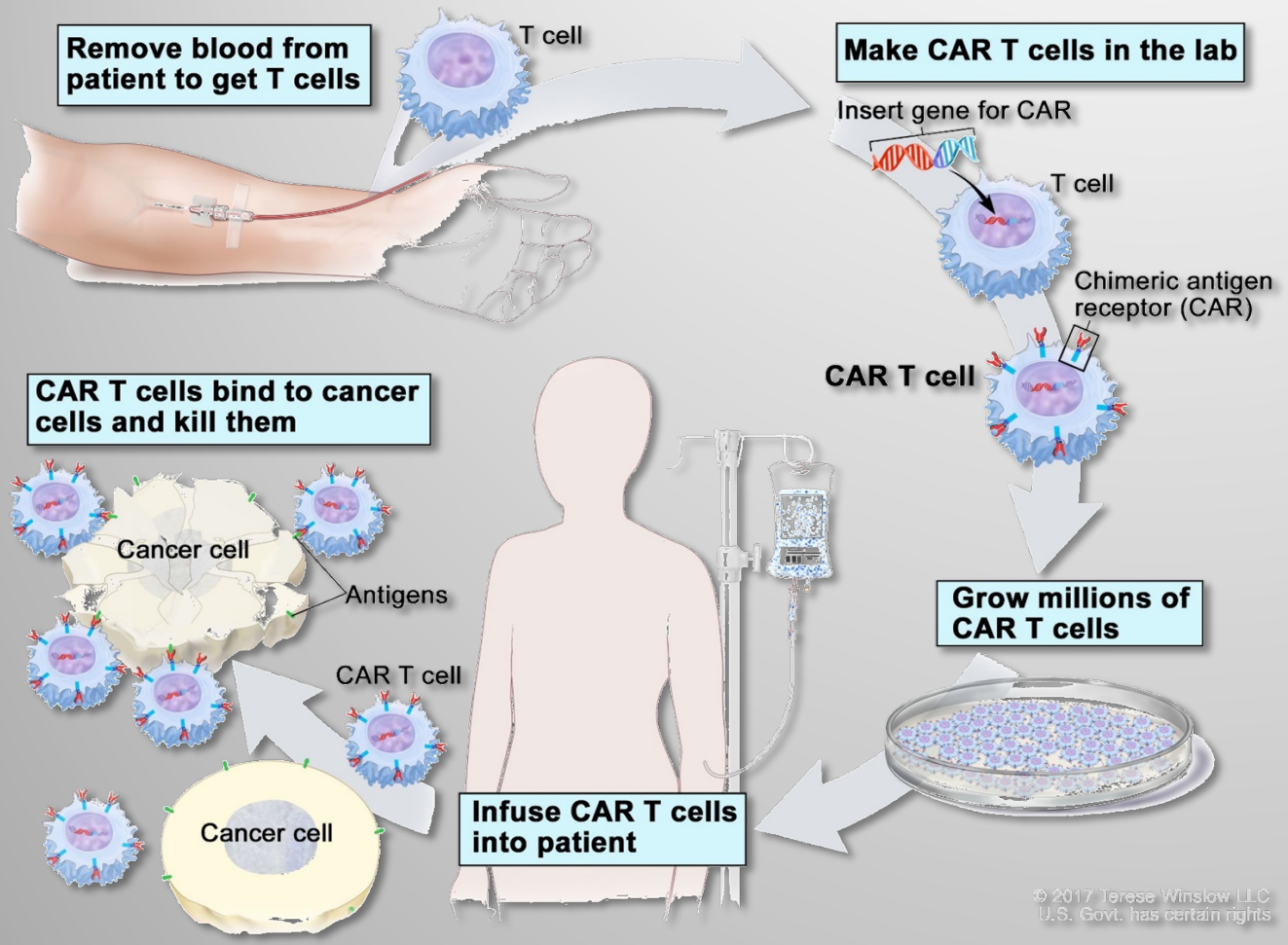

- **19 Cell and Gene products currently approved**
- **500+ cellular therapy trials** on-going
- **>2000 active** cell and gene therapies in development (52% in Oncology)
- The FDA expects to **approve 10 to 20 cell** / gene therapy products per year by 2025
- **CAR-T will cause significant changes in HCT**

***CAR Ts: Next-generation evolution in drug development toward true personalized medicine in oncology***

*Sources: Clinicaltrials.gov; ASCGT Quarterly report 2022*

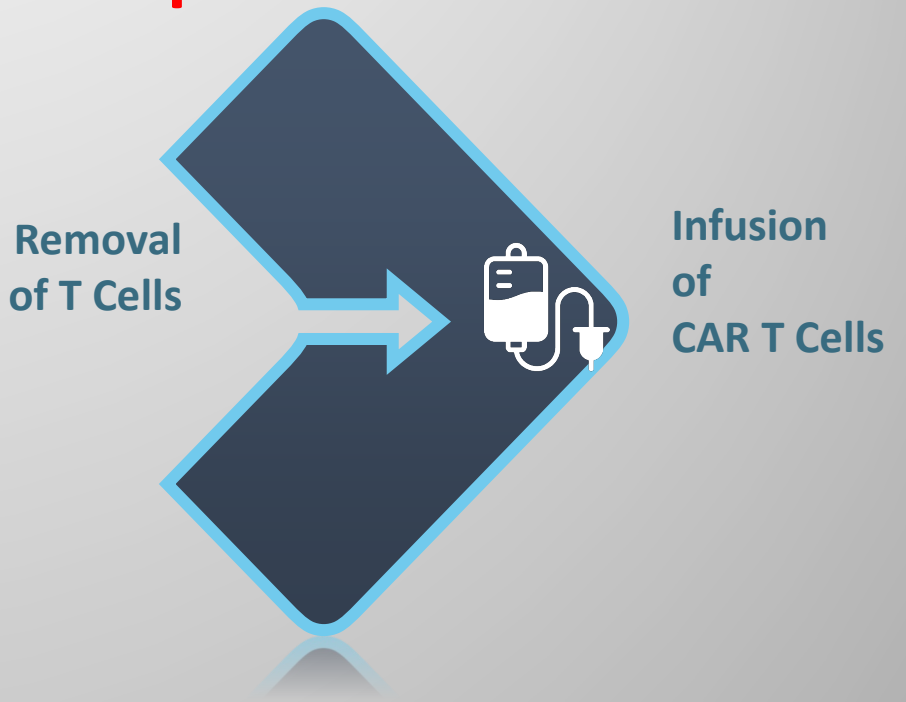
# How are CAR T Cells Manufactured?

**The process to remove T Cells and reinfuse CAR T cells is straight forward occurring on a 21-day horizon.**




**21 Day Process**

**1 product at a time**



# Pace of Product Approval (look at the cost)

| 2017 | 2018 | 2019 | 2020 | 2021 | 2022 | 2023 |
|---|---|---|---|---|---|---|
| KYMRIAH (tisagenlecleucel) Suspension for IV infusion | KYMRIAH (tisagenlecleucel) Suspension for IV infusion | | TECARTUS (brexucabtagene autoleucel) | Breyanzi (lisocabtagene maraleucel) | CARVYKTI (ciltacabtagene autoleucel) | |
| **Acute Lymphoblastic Leukemia**<br>Patients ≤ 25 years old with refractory/relapsed B-cell Acute Lymphoblastic Leukemia (ALL)<br>**Cost - $543,827** | **Large B-Cell Lymphoma**<br>Adult patients with R/R large B-cell lymphoma[1] after ≥2 lines systemic therapy<br>**Cost - $427,047** | | **Acute Lymphoblastic Leukemia**<br>Adult patients with R/R for ALL<br>**Cost - $373,000** | **Large B-Cell Lymphoma**<br>Adult patients with R/R large B-cell lymphoma[2] after ≥2 lines systemic therapy | **Multiple Myeloma**<br>Adult patients with relapsed or refractory multiple myeloma<br>**Cost $465,000** | |
| YESCARTA (axicabtagene ciloleucel) Suspension for IV infusion | | | | YESCARTA (axicabtagene ciloleucel) Suspension for IV infusion | | |
| **Large B-Cell Lymphoma**<br>Adult patients with R/R large B-cell lymphoma after ≥2 lines systemic therapy<br>**Cost - $424,000** | | | | **Follicular Lymphoma**<br>Adult patents with R/R follicular lymphoma after ≥2 lines systemic therapy<br>**Cost - $424,000** | | |
| | | | | Abecma (idecabtagene vicleucel) | | |
| | | | | **Multiple Myeloma**<br>Adult patients with R/R multiple myeloma after ≥4 lines prior therapy[3]<br>**Cost - $457,255** | | |

*Cost of products shown as 2023 prices*

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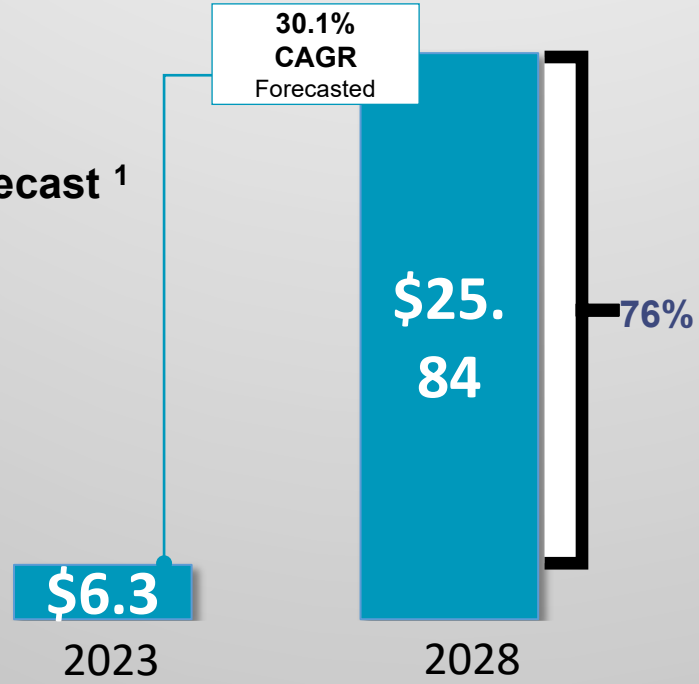
# The Market

**CAR-T Cell Therapy global market value by 2028 is forecasted to be $25.84B with 76% anticipated globally.[1]**

**CAR T Cell Therapy Market Value Forecast [1]**
2023 – 2028; Region Scope: Global;
$ Shown in Billions




Market restraints:

- Lack of skilled professionals (doctors and scientists)
- Cost
- Stringent regulations
- Toxicities
- Concerns about unethical use of gene therapies

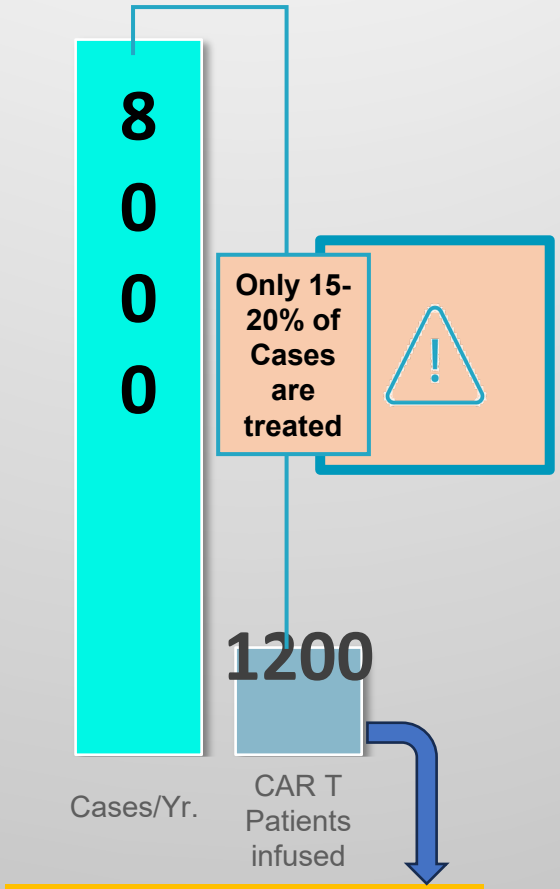
*1 Market Data Forecast: Gene Therapy Market 2023*

# A Major Challenge…

## Patient Access is Limited

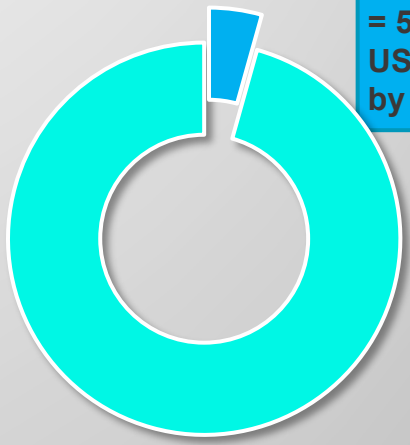
**In the US, CAR T is predominantly offered in the ~200 academic medical centers leaving the ~ 5500 community providers as an untapped care delivery resource for Cell Therapy.**

**1- US Incidence of DLBC and CAR T**




**2- Manufacturing not easily scalable**

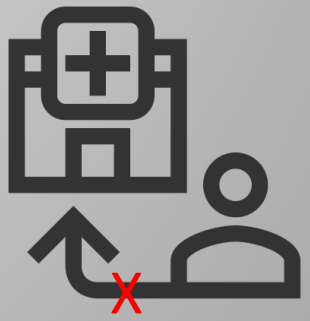
**3- CAR T Cells are available in Academic Medical Centers=~3-5% of health care facilities**



**Academic Medical Centers = 5% of Health Systems in US AMCs, ~3% accredited by FACT**

**Community= 5,500 or 95% of facilities.**

**4- Most oncologists do not refer or administer** CAR T-cell therapy at all



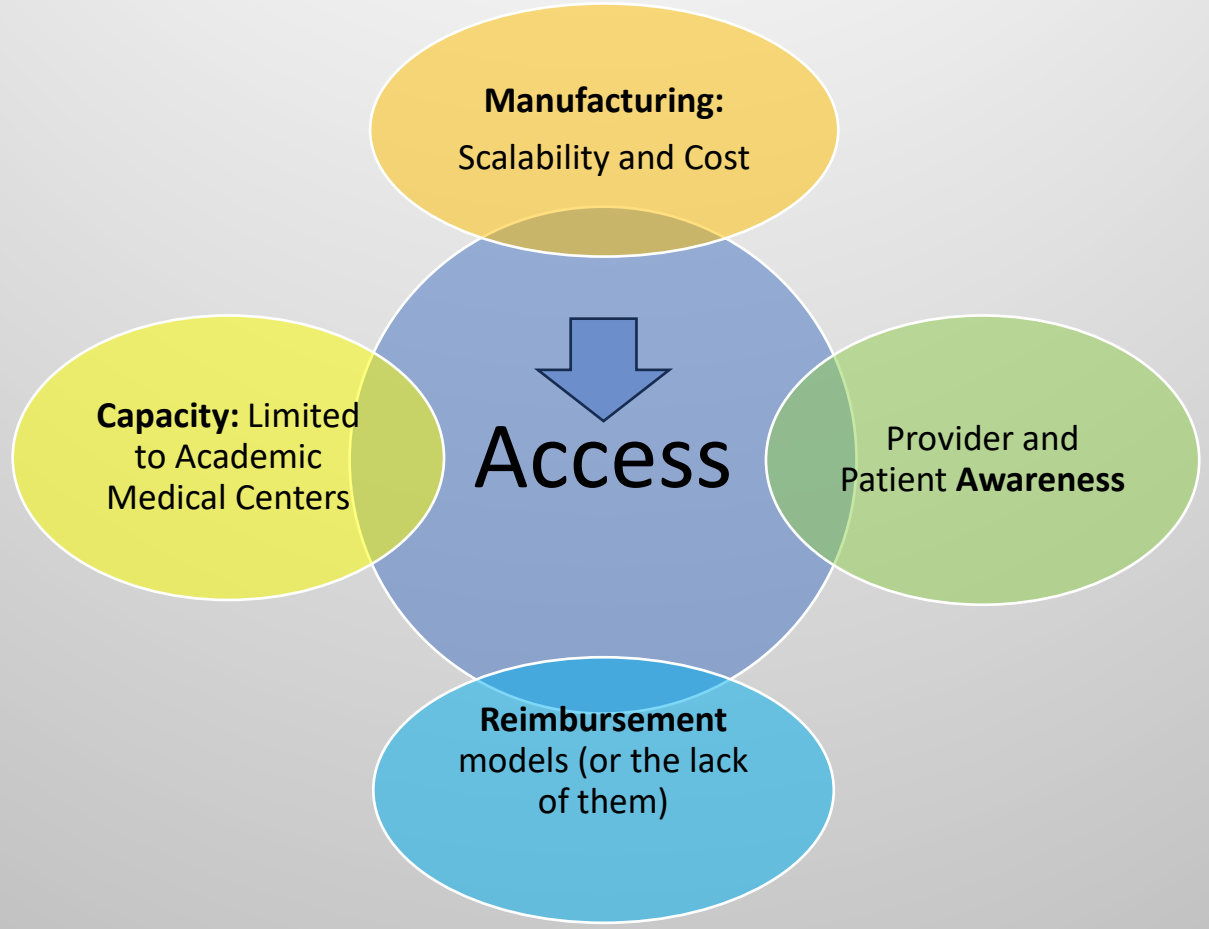
# Global Access

Access to Cell Therapy is even more limited outside of the US due to cost of the products

- US healthcare and clinical research models cannot be exported, as they cannot adjust to the reality of most countries

- Patients from countries outside the US die with cancers that are treatable and potentially curable by cell therapy

# RECAP: BARRIERS TO ACCESS



# What does it take?
# A-Infrastructure and Services
## The 3 Main Categories

**INFRASTRUCTURE AND PERSONNEL:**
- Clinical
- Collection
- Lab/Cell Processing

**IMPLEMENTATION/TRAINING:**
- Clinical Operations
- Quality

**REGULATORY SUPPORT:**
- Clinical Research
- Commercialization

A VERY SPECIFIC SET OF SKILLS

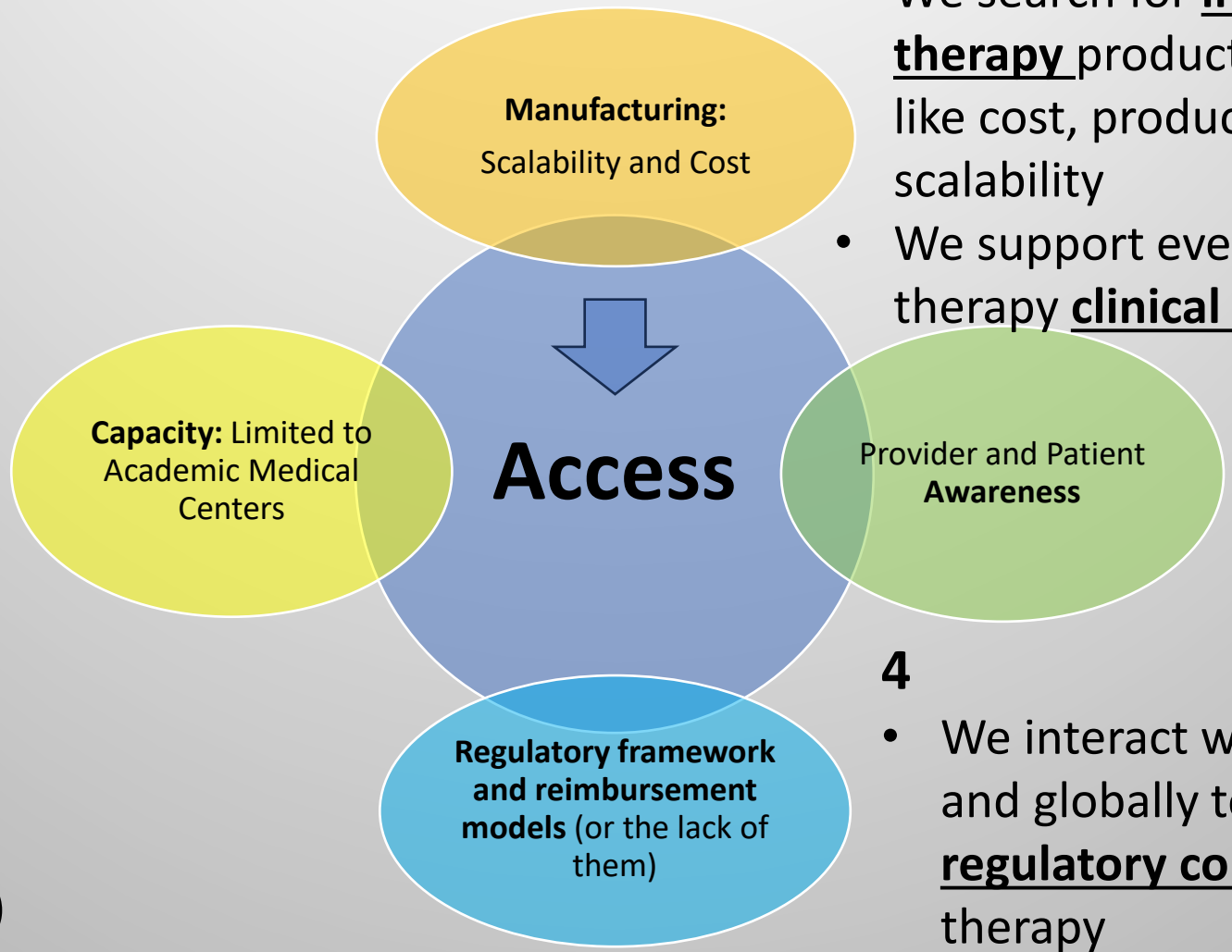
# What does it take?
# B-The Right Product

- Manufacturing:
  - Simpler
  - Faster
  - Less costly
  - Scalable
  - Exportable
- Efficacy:
  - Stemness, persistence
- Toxicity profile
- Better and simplified resource management strategies associated to both manufacturing and delivery operations

# How We Do It

**1**

- **Consulting** services to US and globally to establish new community Cellular Therapy programs and enhance and scale existing ones.
- **Products** needed to that end (SOP, Quality Management Plans, etc)




**2**

- We search for **innovative cell therapy** products that address issues like cost, production time and scalability
- We support every aspect of cellular therapy **clinical research**

**3**

- Provide education

**4**

- We interact with stakeholders in US and globally to contribute the **regulatory complexities** of cellular therapy

# Cellular therapy is a new form of treatment, with its own clinical, operational, quality and regulatory standards that differ from those in the pharmaceutical industry

1- Clinical: Requires very specific knowledge of adverse reactions that almost exclusively occur in cellular therapy

6- Continuous innovation in cell and gene therapy products

3- Quality: All processes in cellular therapy, from the patient to the lab and back to the patient must follow the highest quality standards

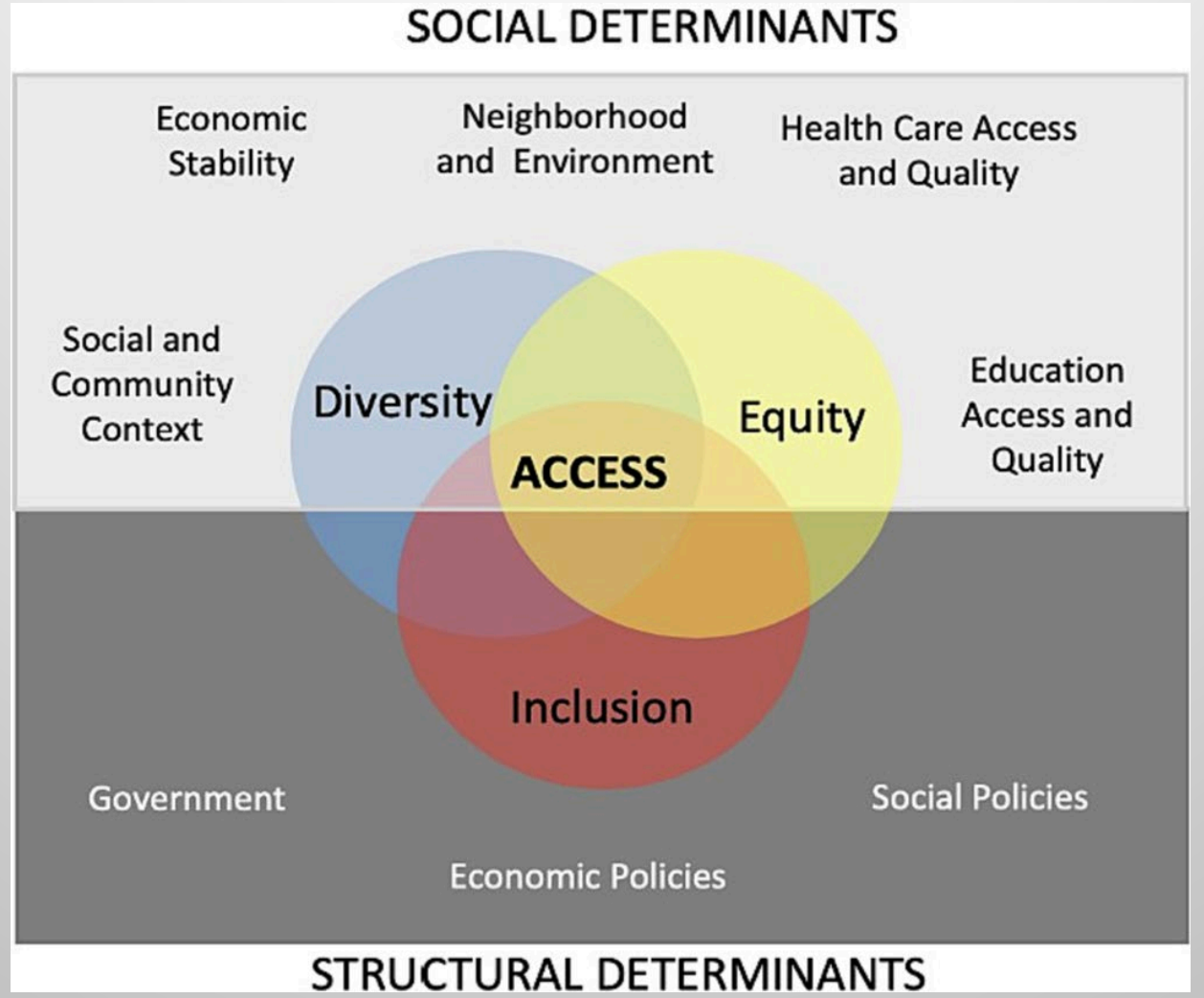

4- Evolving regulatory environment

2- Operational: The complexity in the manufacturing process, including cell collection, shipping cells for genetic engineering, etc. requires a team of people with specific training for each of these steps

5- Evolving financial and reimbursement structure and new investment opportunities in health care innovation

# CONTEXTO






Odstrcil, Couriel. Blood Reviews 63 (2024) 101136


QUESTIONS?