Common Applications of Cell Therapy in Society

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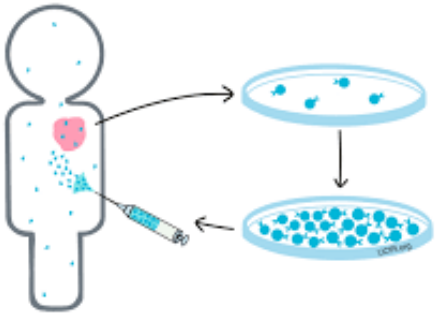
Beyond Wound Repair: Societal Frontlines of Cell-Based Therapies

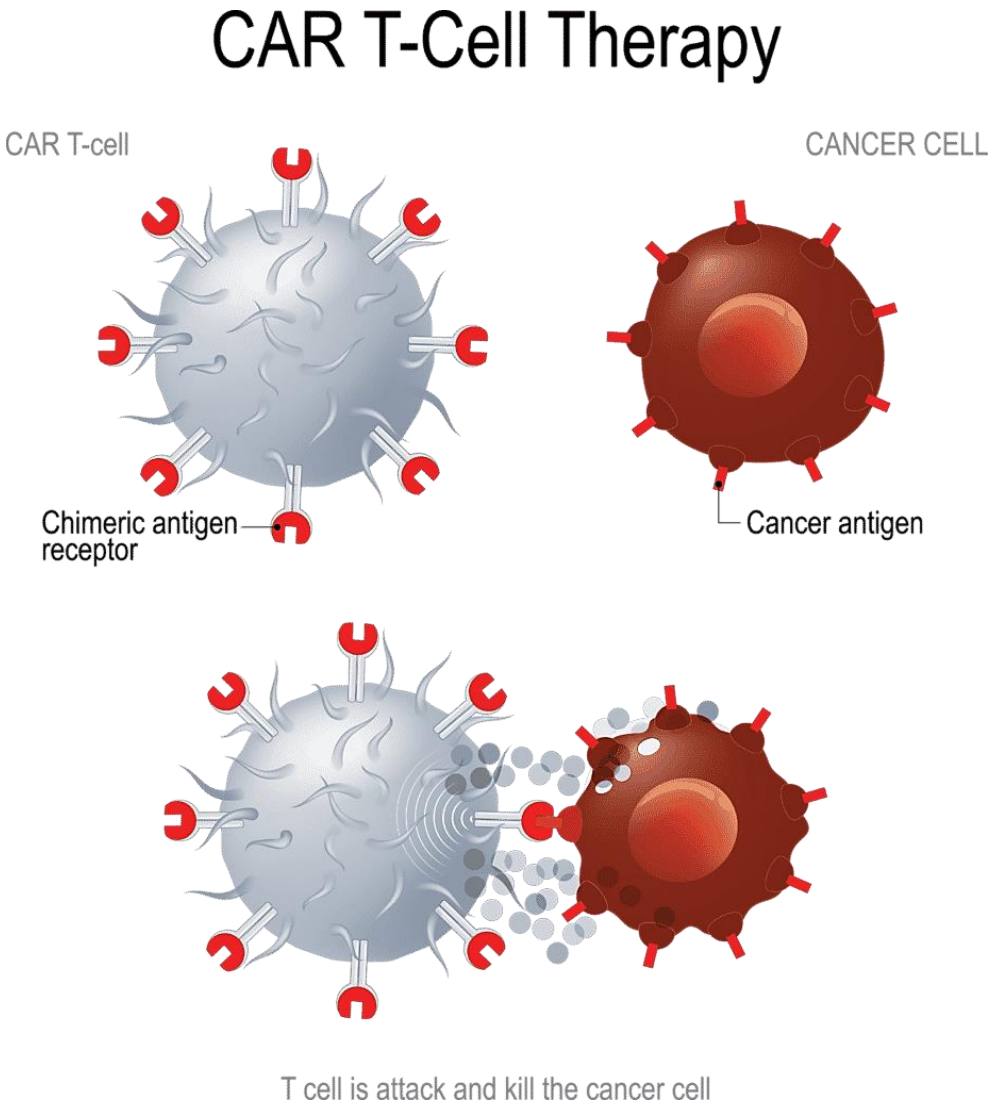
CAR-T Cells – A Paradigm Shift in Oncology (Fighting Cancer with Living Drugs)

- CAR_T cell therapy is a revolutionary treatment where a patient's T-cells are genetically modified to recognize and kill cancer cells.
- Clinical trials show **83% remission rates** in relapsed pediatric acute lymphoblastic leukemia (ALL).
- CAR-T is FDA-approved for:
  - ✓ B-cell lymphomas (*Yescarta*)
  - ✓ Acute lymphoblastic leukemia (*Kymriah*)
  - ✓ Multiple myeloma (*Abecma*)
- It has reshaped the prognosis for patients who previously had no effective treatment options



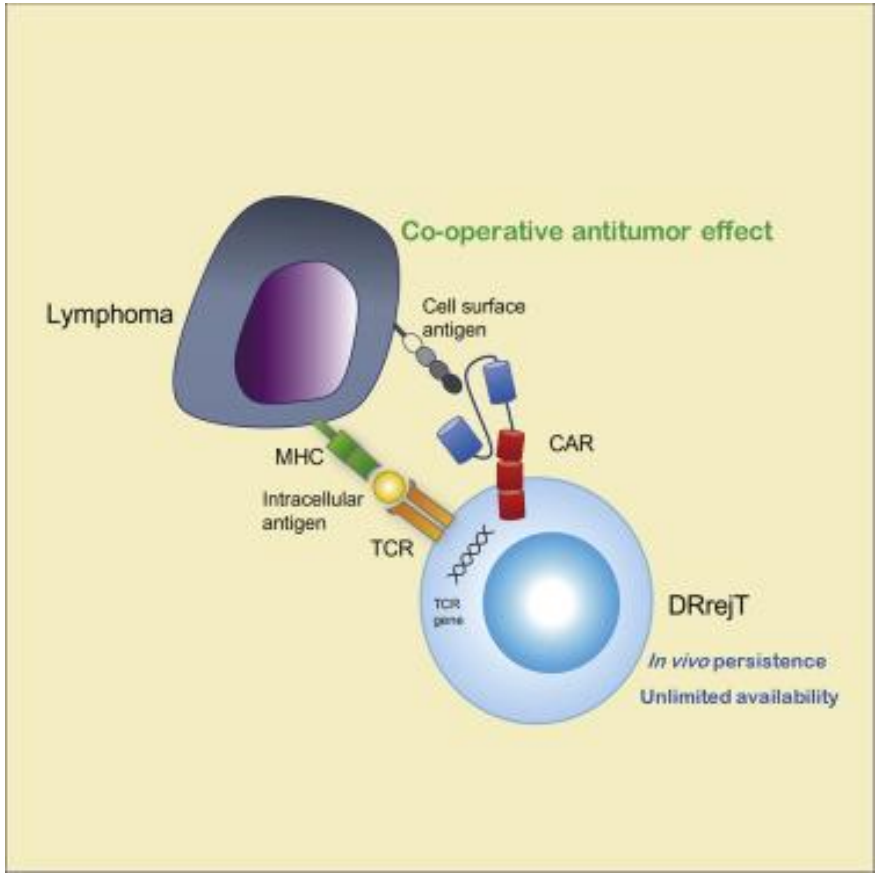
Off-the-Shelf CAR-T – Expanding Access
(Allogeneic Immunotherapy: Ready When Needed)

- Allogeneic iPSC-derived CAR-T therapies are being developed to overcome delays and variability in autologous CAR-T.
- Benefits include:
  - ✓ **Immediate availability** for critically ill patients.
  - ✓ Elimination of T-cell harvest from the patient.
  - ✓ **Reduced production costs** by up to 60%.
- Trials with "universal donor" cells have shown **comparable efficacy with fewer logistics issues**



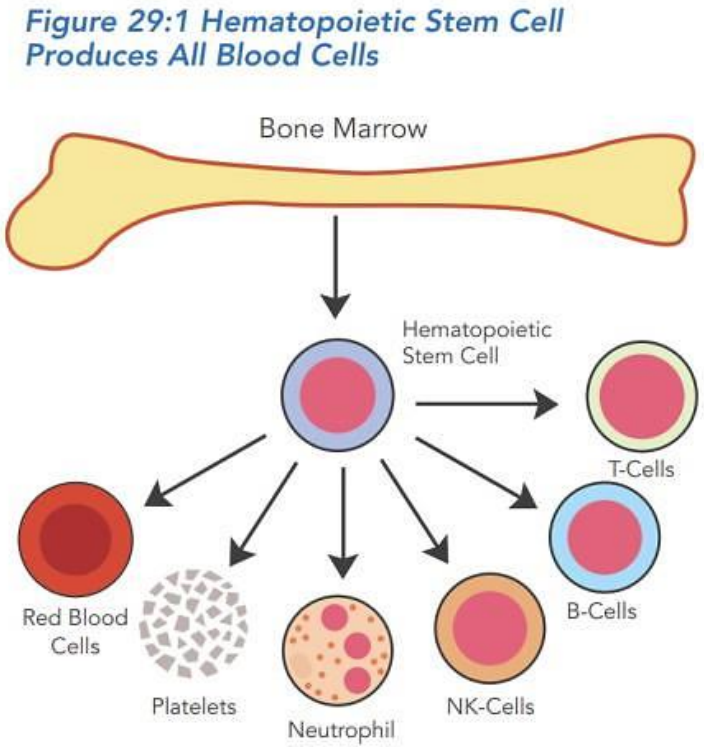
iPSC-Derived CAR-T – A Scalable Future (Using Reprogrammed Cells for Mass-Produced Immunity)

- iPSC-drived CAR-T cells offer:
  - ✓ Unlimited, renewable T-cell source.
  - ✓ Genomic editing (e.g., CRISPR/Cas9) before T-cell differentiation.
  - ✓ Consistency across batches, reducing clinical variability.
- This makes it feasible to **mass-produce cell therapies** similar to traditional drugs



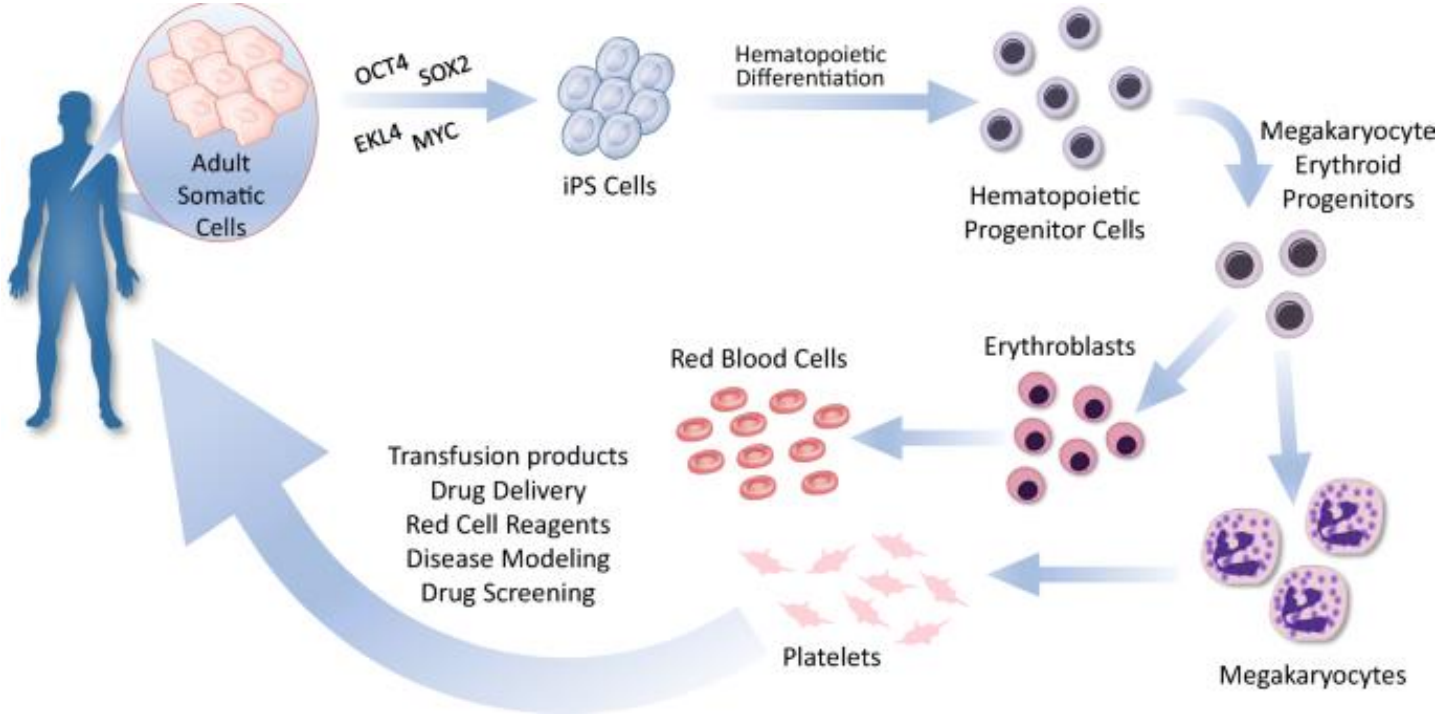
Hematopoietic Stem Cell Transplants (HSCT) (*The Backbone of Blood Regeneration)*

- HSCT is a well-established therapy, with over **50,000 annual procedures worldwide** for leukemia, lymphoma, and bone marrow failure.
- It restores:
  - ✓ Hematopoiesis (blood formation)
  - ✓ Immune competence
- Conditioning (e.g., chemo/radiation) and graft matching are key challenges.
- Sources include bone marrow, peripheral blood, and **umbilical cord blood**



iPSC-Derived Blood Cells (Personalized Hematopoiesis Without Donors)

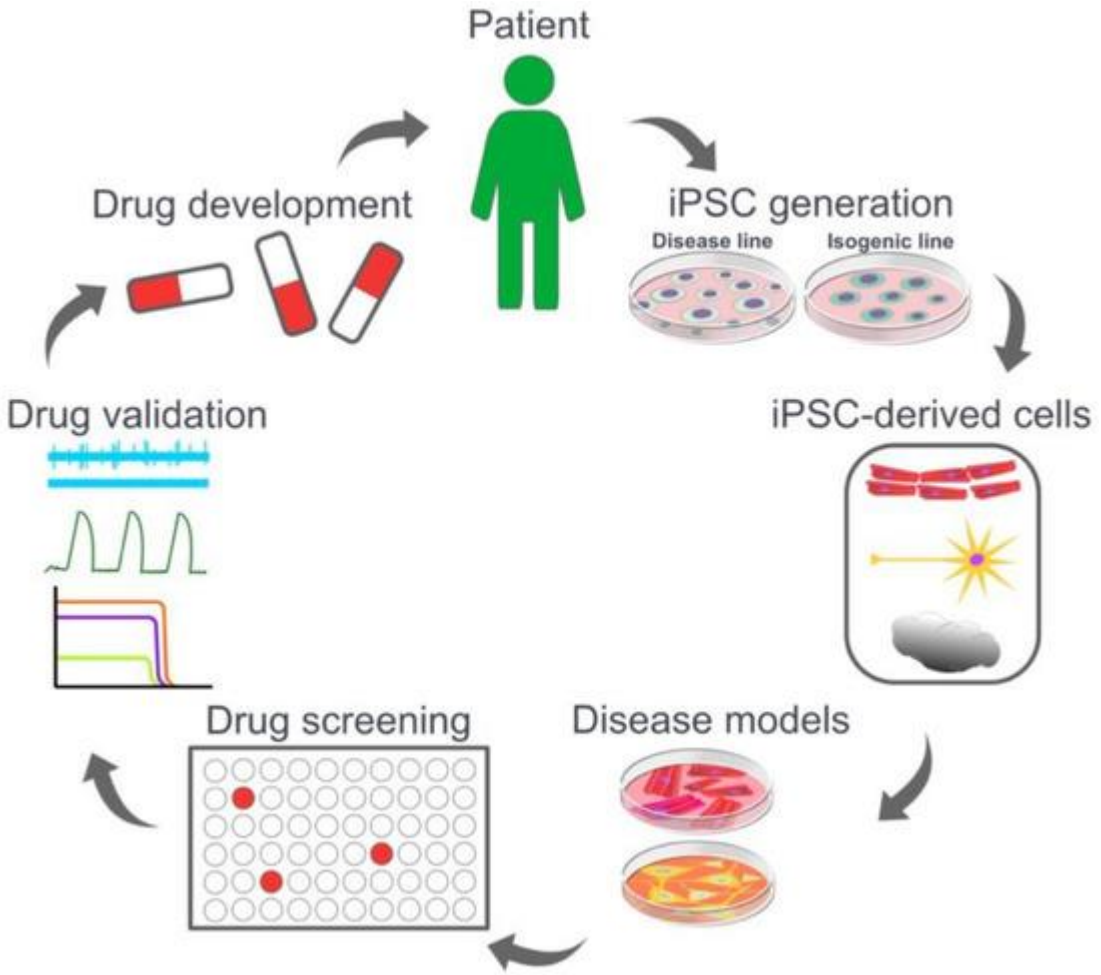
- iPSCs can be differentiated into CD34+ hematopoietic progenitors.
- Enables:
  - ✓ Patient-specific blood cells for transplant
  - ✓ Disease modeling for blood disorders
- Reduces dependency on donor registries and HLA matching



iPSCs in Disease Modeling & Gene Repair

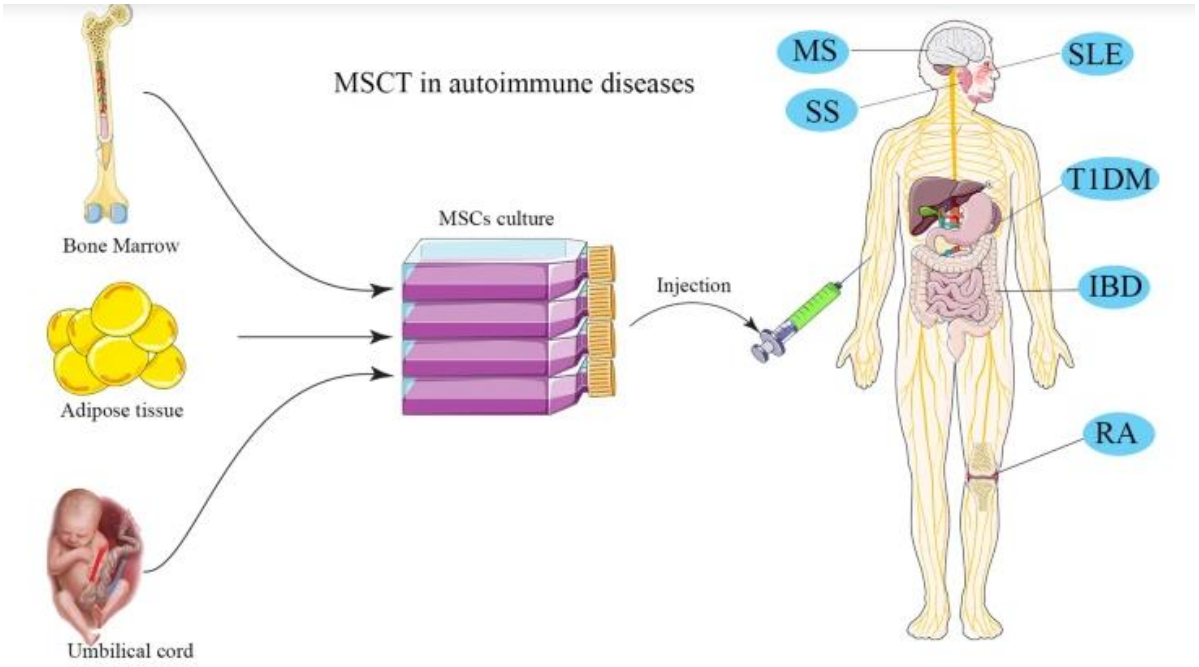
(Precision Medicine in a Dish)

- iPSC platforms are used to replicate patient-specific disease states—such as Parkinson's, ALS, diabetes—for testing drugs without human trials.
- iPSCs allow **gene correction via CRISPR**, then redifferentiation into healthy cells for potential autologous transplantation.
- This makes them powerful tools for both **research and therapeutic development**



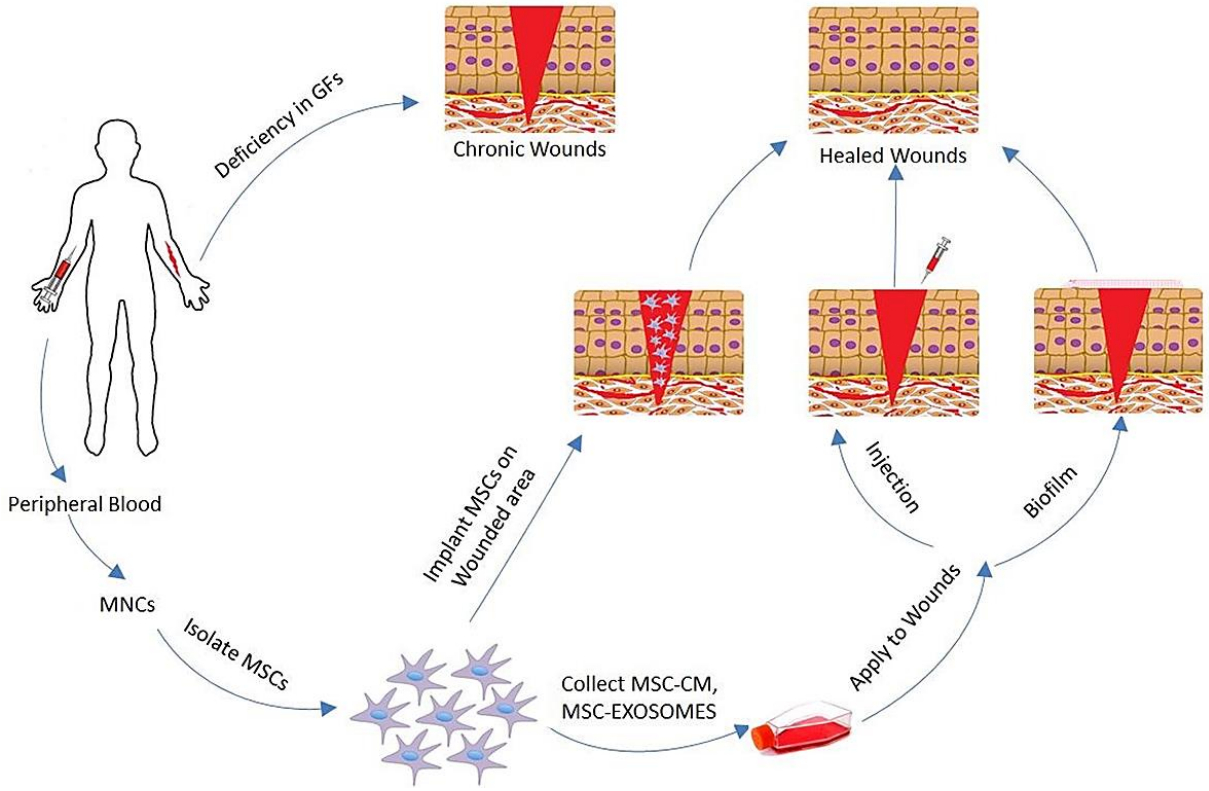
Neurological and Autoimmune Applications (Cell Therapies for the Brain and Immune System)

- Trials show promise using:
  - ✓ iPSC-derived neurons for **ALS and spinal cord injuries**
  - ✓ **Regulatory T cells (Tregs)** for autoimmune diseases like MS, Type 1 diabetes, and GVHD.
- T-iPSC strategies support generation of helper and Treg lineages to dampen harmful immune responses



MSCs in Chronic Wound Healing

- ✓ **Diabetic Foot Ulcers**:
  - MSCs injected at wound edges stimulate local angiogenesis, reduce inflammation, and improve oxygen delivery.
  - Clinical trials report faster healing, fewer infections, and reduced hospitalization duration.
- ✓ **Burns**:
  - MSCs improve epithelial cell growth and dermal matrix reconstruction.
  - Delivery via scaffolds (e.g., fibrin sprays, hydrogels) improves cell viability.
- ✓ **Pressure Ulcers** (bedsores):
  - BM-MSCs reduce chronic inflammation and promote dermal regeneration.
  - One study showed **40% wound size reduction in 20 weeks** using MSCs plus fibrin spray.



MSCs After Myocardial Infarction

✓ **Cardiac Effects**:

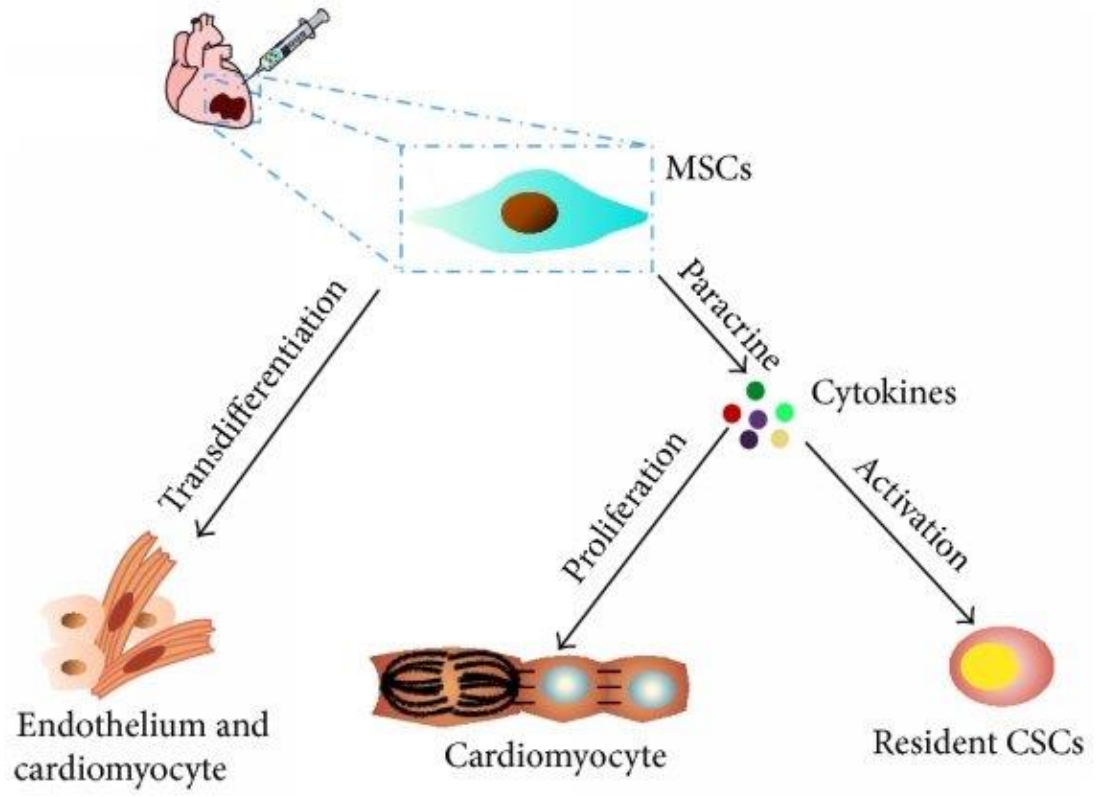
- ↑ Left ventricular ejection fraction (LVEF) by **3.8%**, ↓ scar tissue.
- Promotes **angiogenesis** and **reduces cardiomyocyte apoptosis**.

➢ **Immunomodulation**:

- MSCs shift macrophages from M1 (inflammatory) to M2 (repair phenotype).
- They also increase secretion of IL-10, TGF-β — reducing cardiac inflammation.

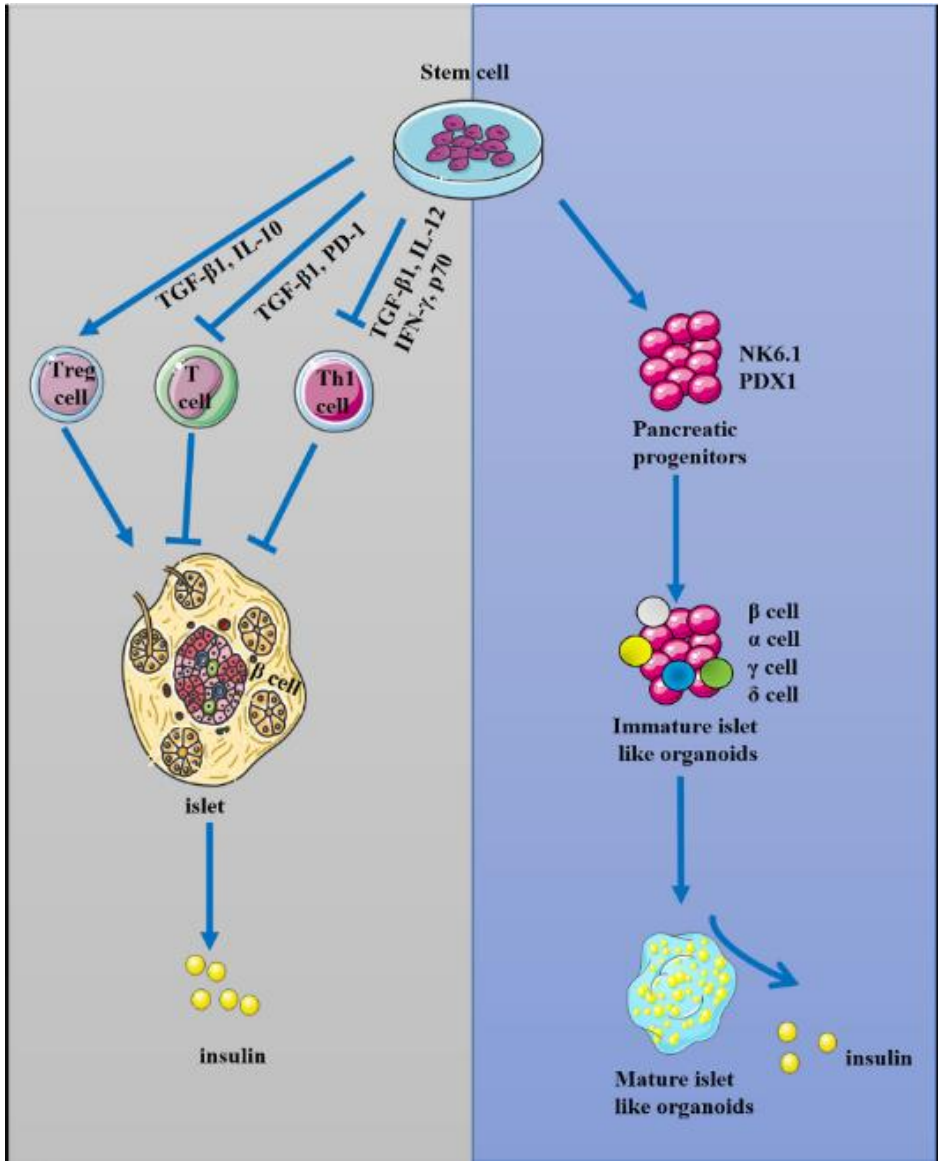
➢ **Clinical Significance**:

- Improved cardiac function sustained over 6–24 months post-therapy.



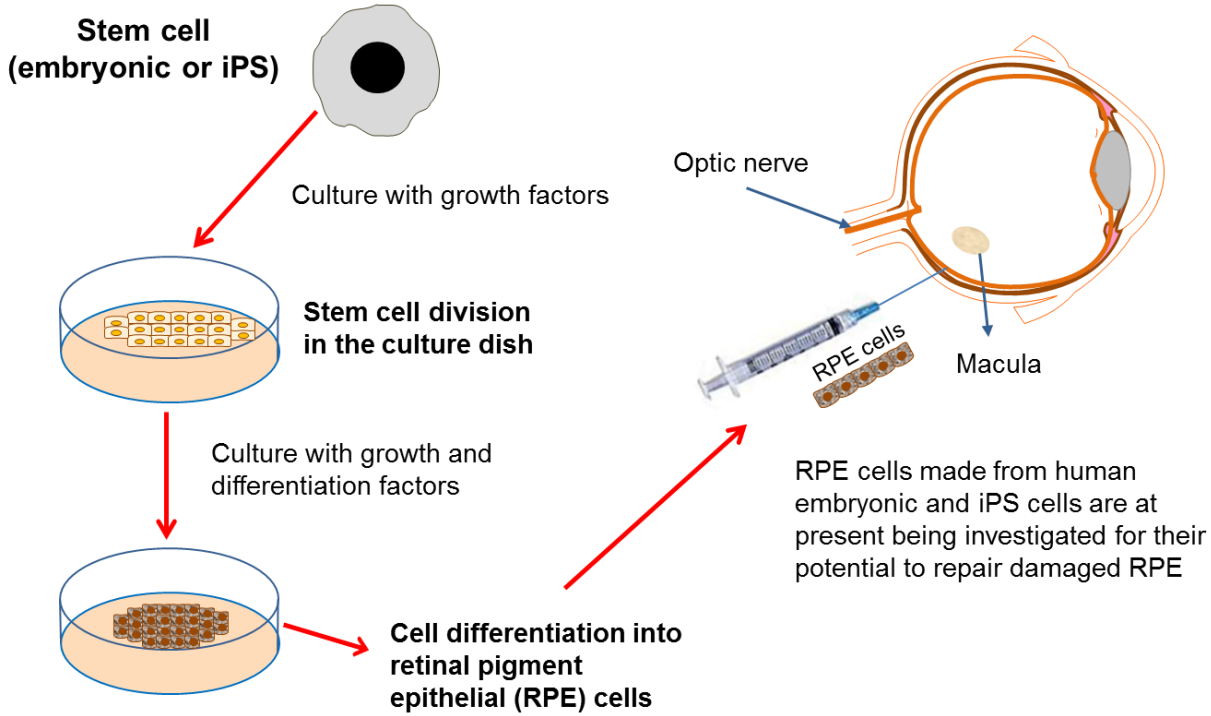
MSCs in Type 1 Diabetes

- ✓ **β-Cell Support or Replacement**:
  - Under lab conditions, MSCs can express insulin when stimulated with transcription factors.
  - In animals, this has partially restored insulin levels.
- ✓ **Tissue Regeneration**:
  - MSC-secreted VEGF, IGF-1, HGF → support existing β-cell proliferation.
  - MSC exosomes carry anti-apoptotic and anti-inflammatory miRNAs.
- ✓ **Immune Regulation**:
  - Suppress autoreactive T-cells, upregulate Tregs.
  - Delay or prevent autoimmune attack.



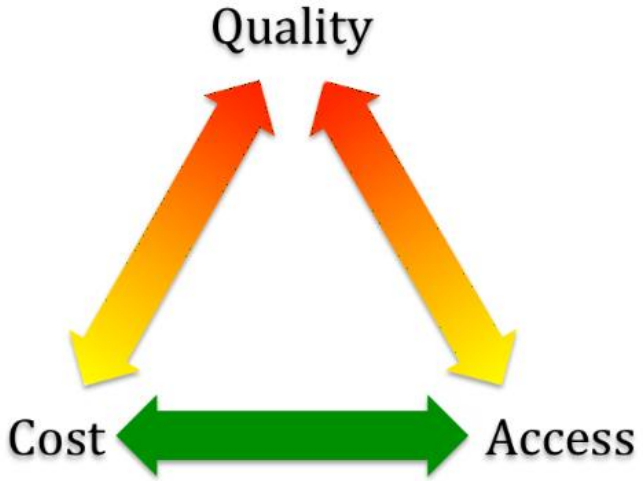
Retinal Diseases and Vision Restoration

- ✓ **iPSC/ESC-Derived RPE Cells**:
  - Implanted under retina (via patches) to treat AMD.
  - Showed **partial vision recovery** and stabilization in early-phase trials.
- ✓ **MSC Use in Eyes**:
  - Instead of cell replacement, MSCs modulate **retinal inflammation** and release neurotrophic factors.
  - Helps **slow degeneration** in glaucoma, retinitis pigmentosa, and AMD.
- ✓ **Challenges**:
  - Still experimental.
  - Larger RCTs are needed to confirm long-term benefits.

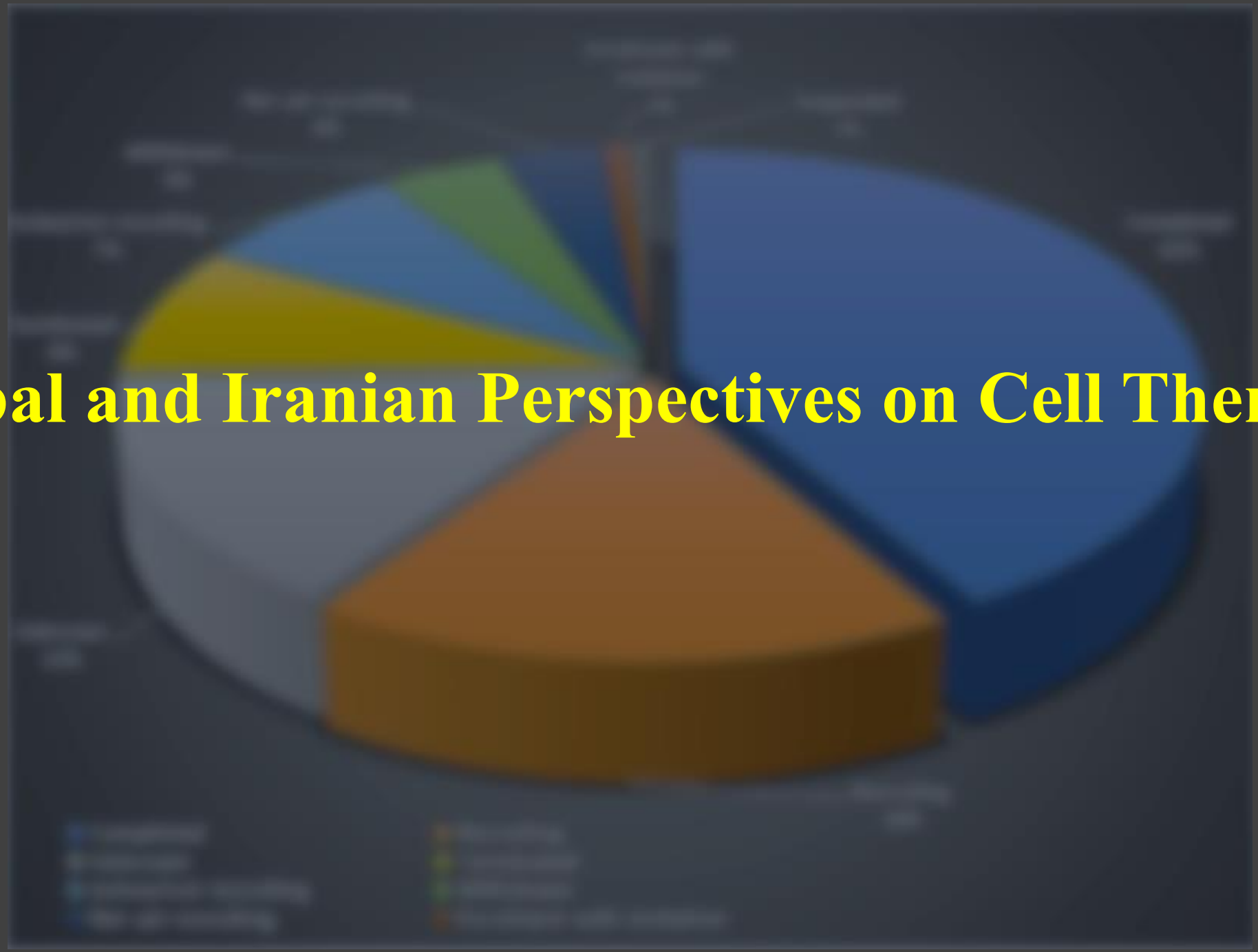


Cost and Access (Is Cell Therapy Economically Sustainable?)

- CAR-T therapy prices exceed **$475,000 per dose** (e.g., *Kymriah*).
- iPSC-based therapies may cut costs by enabling mass production with uniform quality.
- Insurance systems are struggling to keep up, highlighting the need for new **reimbursement models**.

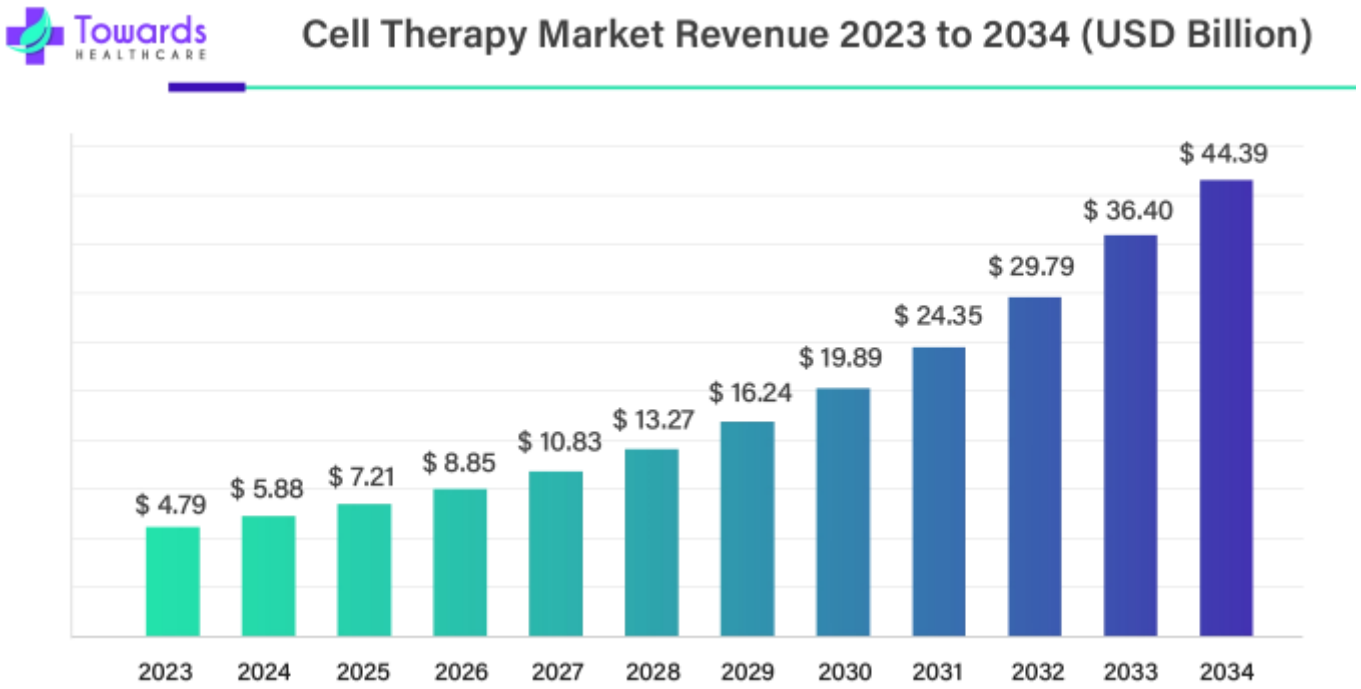


Global and Iranian Perspectives on Cell Therapy



Market Size & Growth

- The global cell therapy market size was estimated at US$ 7.21 billion in 2025 and is projected to grow to US$ 44.39 billion by 2034, rising at a compound annual growth rate (CAGR) of 22.69% from 2024 to 2034.



Major Key Insights of the Cell Therapy Market

- North America dominated the market share by 59% in 2023.
- Asia Pacific is expected to grow at a significant rate during the forecast period.
- By therapy type, the autologous therapy segment dominated the cell therapy market in 2023.
- By therapeutic area, the oncology segment held the largest share of the market in 2023.

Therapeutic Areas Using Cell Therapies

- **Oncology** accounts for roughly **37–40%** of usage—primarily CAR-T and TCR therapies for blood cancers (leukemia, lymphoma), increasingly applied in solid tumors
- **Cardiovascular diseases** represent about **20%**, with stem-cell therapies aimed at regenerating heart tissue post-myocardial infarction or heart failure
- **Musculoskeletal disorders** (≈15%) such as osteoarthritis and orthopedic injuries are treated with cell therapy for cartilage/joint repair
- **Neurological disorders** (Parkinson's, Alzheimer's, spinal cord injuries) and **ophthalmology** (macular degeneration) are emerging (~10–15%)
- Other applications include **autoimmune and infectious diseases**, though smaller in scale but showing high growth potential

Cell Therapy in Iran: Progress & Applications (National Activity & Statistics)

- Iran ranks **12th globally and 1st in West Asia** in biotechnology, with over **60% of exports** from knowledge-based companies tied to biotech, including cell therapy .
- A national project began in late **2022**, first clinically applying **CAR-T therapy for chemotherapy-resistant pediatric leukemia**, marking Iran's entry into gene-edited immunotherapies .
- Since **2018**, Iran houses **West Asia's first mass-production stem cell facility** (Cell Tech Pharmed), producing standardized stem-cell products like *Monocell*, *Ricollersal*, *Rhinooderm* for cardiovascular, skin, and musculoskeletal conditions
- The government plans to establish Iranian **cell therapy centers abroad**, promoting technology exportation and regional collaborations

Diseases Treated (Iran-Specific)

- **Leukemia (pediatric)** with experimental CAR-T therapy in clinical trials .
- **Cardiac diseases**, **orthopedic or musculoskeletal disorders**, and **dermatological conditions** (e.g., skin regeneration, arthritis) using cell therapy products like Monocell and Rhinooderm .
- Likely expanded toward vascular, skin, knee-arthritis therapies through domestic stem-cell derived products.

Companies & Institutions in Iran & Globally

Institutional & Industrial Landscape in Iran

- **Royan Institute / Royan Stem Cell Technology Co.** (under ACECR): pioneered Iran's first embryonic stem cell line (2003), private cord-blood banking (2005), and cell therapy pre-hospital services since 2011 .
- **Cell Tech Pharmed** (subsidiary of Barkat Pharmaceutical Group & Royan Institute): operates Iran's first large-scale stem cell production facility (est. 2014); provides cell therapy products (Monocell, Ricollersal, Rhinooderm) targeting cardiovascular, skin, joint conditions .
- **Barkat Pharmaceutical Group**: umbrella company supplying peptide-based and cell therapies; supports oncology, MS, blood diseases, cardiovascular, skin, skeletal disease therapies.

Leading International Cell Therapy Firms (global context)

- **Lineage Cell Therapeutics** (USA): developing multiple allogeneic cell products such as OpRegen® for dry age-related macular degeneration, OPC1 for acute spinal cord injury, VAC2 dendritic-cell therapy for non-small cell lung cancer, auditory & photoreceptor progenitor therapies.
- **Kite Pharma** (Gilead subsidiary): early approved CAR-T therapies for B-cell lymphoma and other hematologic malignancies; global leader in hematologic oncology cell therapy.
- International pharma giants (Novartis, J&J, Bristol-Myers Squibb) actively advancing CAR-T therapies and reducing manufacturing time to broaden patient access.
- **Vertex's Casgevy**: CRISPR-based gene-edited autologous cell therapy approved in US, UK, EU for sickle cell disease and beta thalassemia (~35,000 eligible patients globally).

References:

- Revolutionizing Cancer Treatments through Stem Cell-Derived CAR T Cells for Immunotherapy: Opening New Horizons for the Future of Oncology
- Advances in Adoptive Cell Therapy Using Induced Pluripotent Stem Cell-Derived T Cells
- Induced Pluripotent Stem Cells (iPSCs) Provide a Potentially Unlimited T Cell Source for CAR-T Cell Development and Off-the-Shelf Products
- Hematopoietic Stem Cell Transplantation
- Engineered T cells from induced pluripotent stem cells: from research towards clinical implementation
- Utility of iPSC-Derived Cells for Disease Modeling, Drug Development, and Cell Therapy
- Regeneration of antigen-specific T cells by using induced pluripotent stem cell (iPSC) technology
- Combining the induced pluripotent stem cell (iPSC) technology with chimeric antigen receptor (CAR)-based immunotherapy: recent advances, challenges, and future prospects
- Cell Therapy Market Size, Trends, Growth Drivers and Strategic Forecasts
- Iran planning to launch cell therapy centers in neighboring countries


Thank you
for your
attention