The Gates Center

FOR REGENERATIVE MEDICINE

PRESENTATION TO

House Health and Human Services Committee

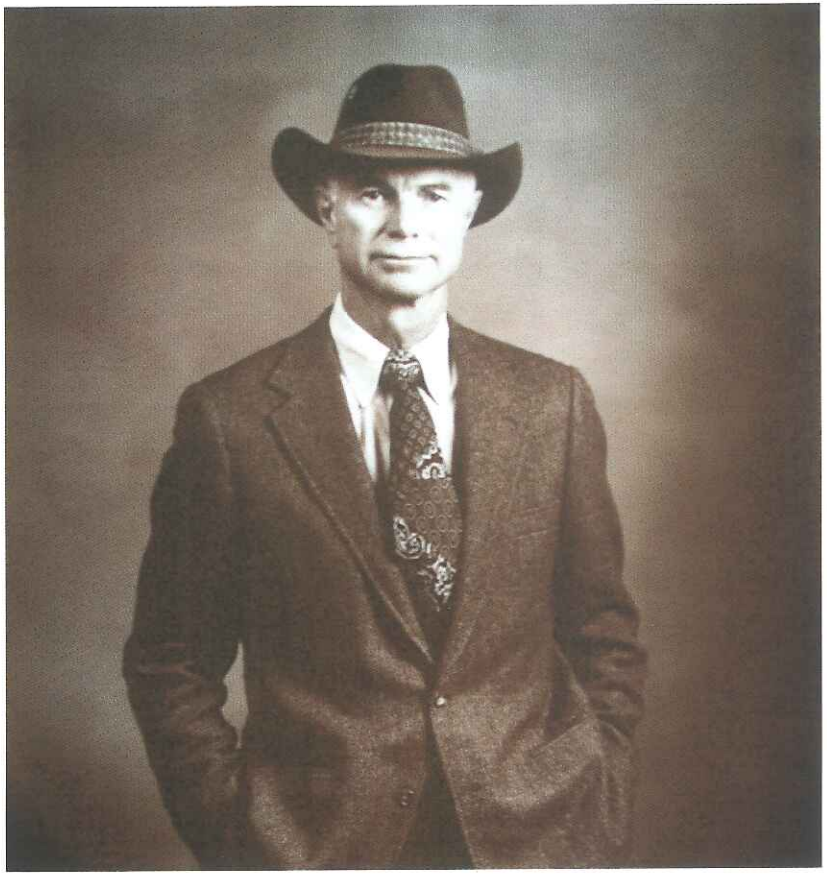
3.10•2015

Gates Center for Regenerative Medicine

Attachment A

Charles C. Gates

A visionary who saw the potential of stem cell research



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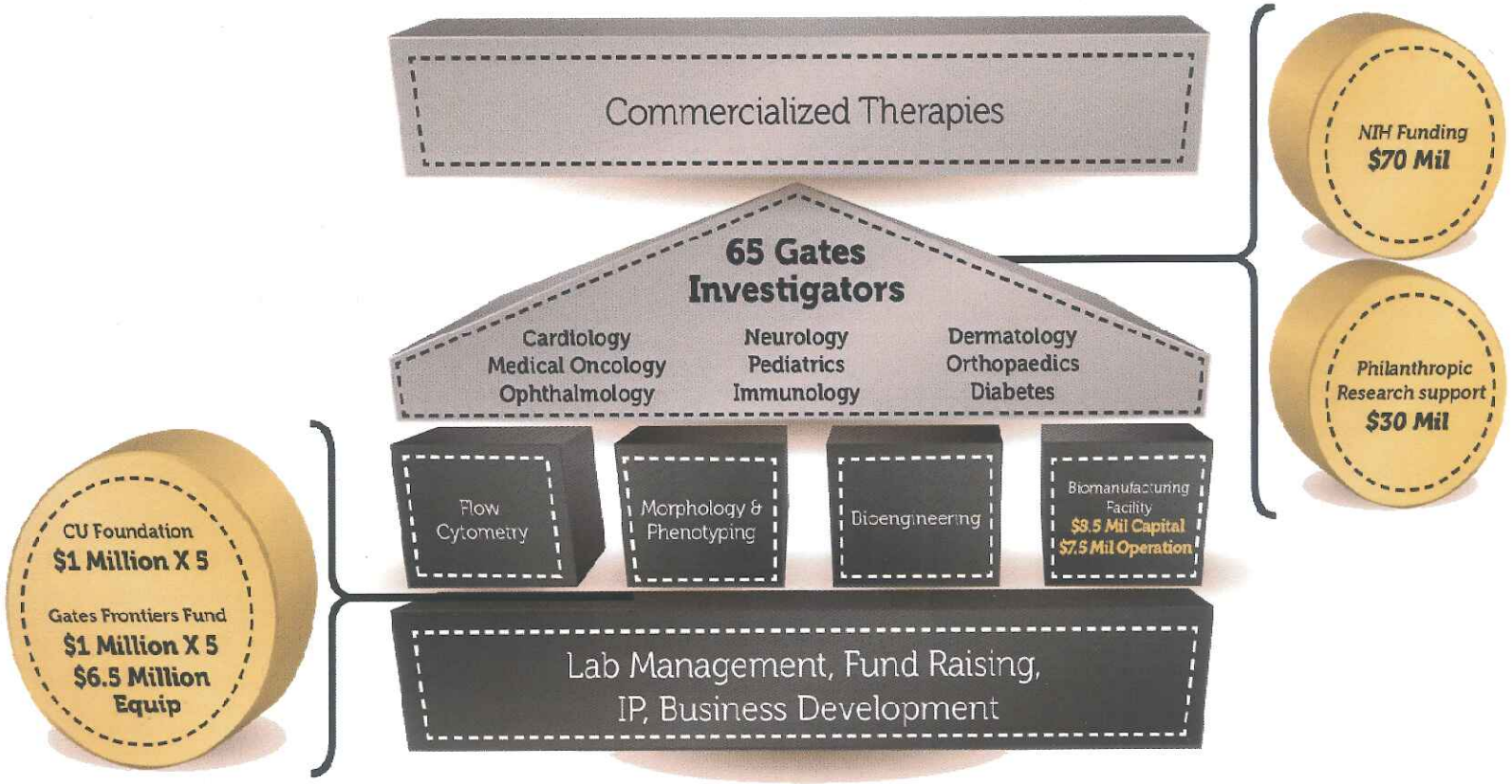
An Investment In The Future

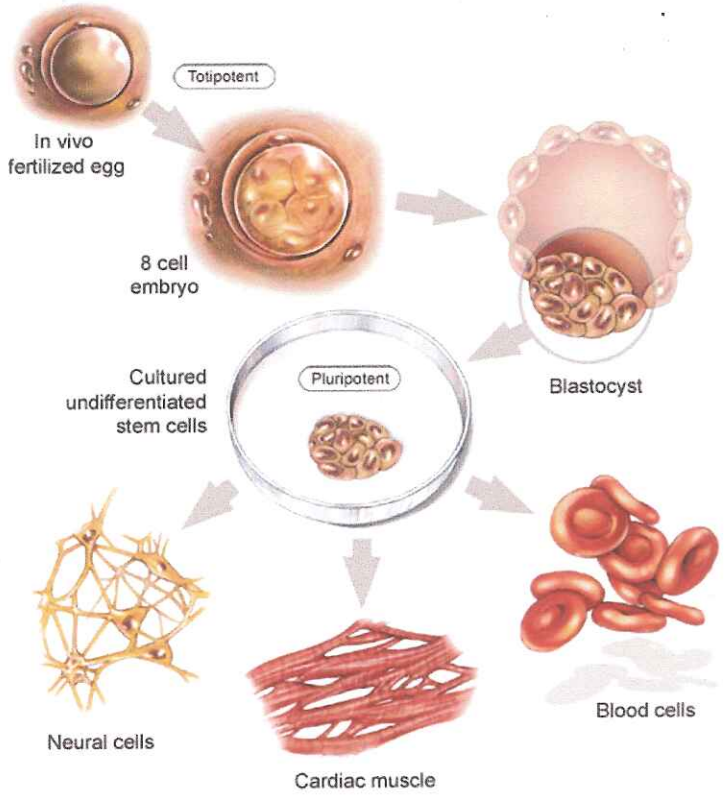


The Major Focus of our Stem Cell Center
Adult Stem Cells
Not Embryonic Stem Cells

WHY?

Embryonic Stem Cells

Potential to Generate All Tissue Types



Concerns: Ethical Issues and Restricted Federal Funding

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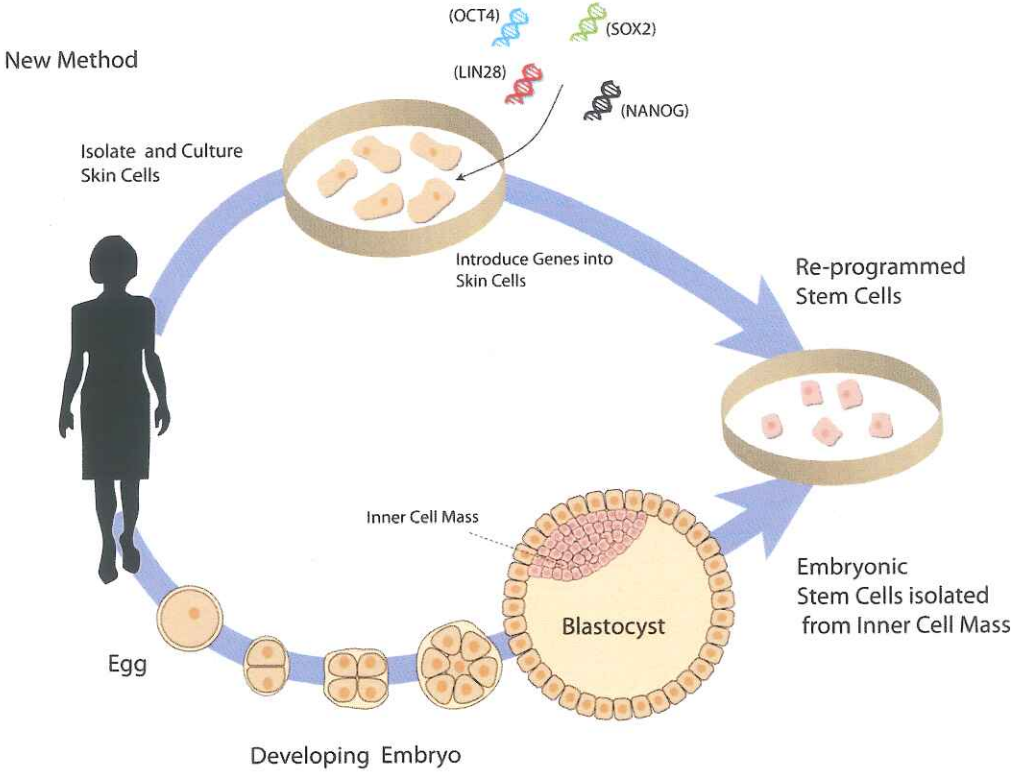
Induced Pluripotent Stem (iPS) cells

Reprogramming Adult Human Skin Cells Into Embryonic-like Stem Cells



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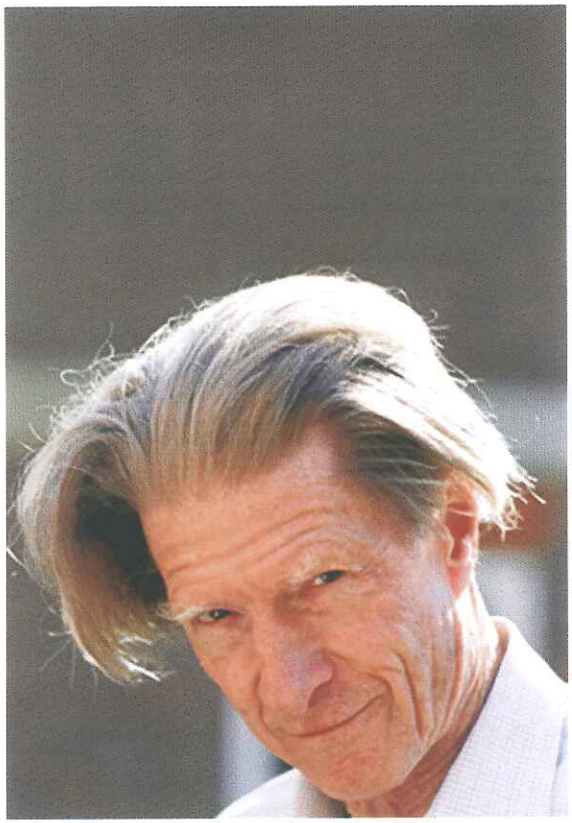
The Nobel Prize in Medicine 2012

Sir John B. Gurdon



Shinya Yamanaka

A Glimpse Into the Future

HOW STEM CELLS MAY CHANGE THE FACE OF MEDICINE

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Induced Pluripotent Stem (iPS) Cells

We are attempting to correct the genetic defect using iPS cells generated from Adam's skin cells

In collaboration with the Epidermolysis Bullosa Center of Excellence at Children's Hospital Colorado

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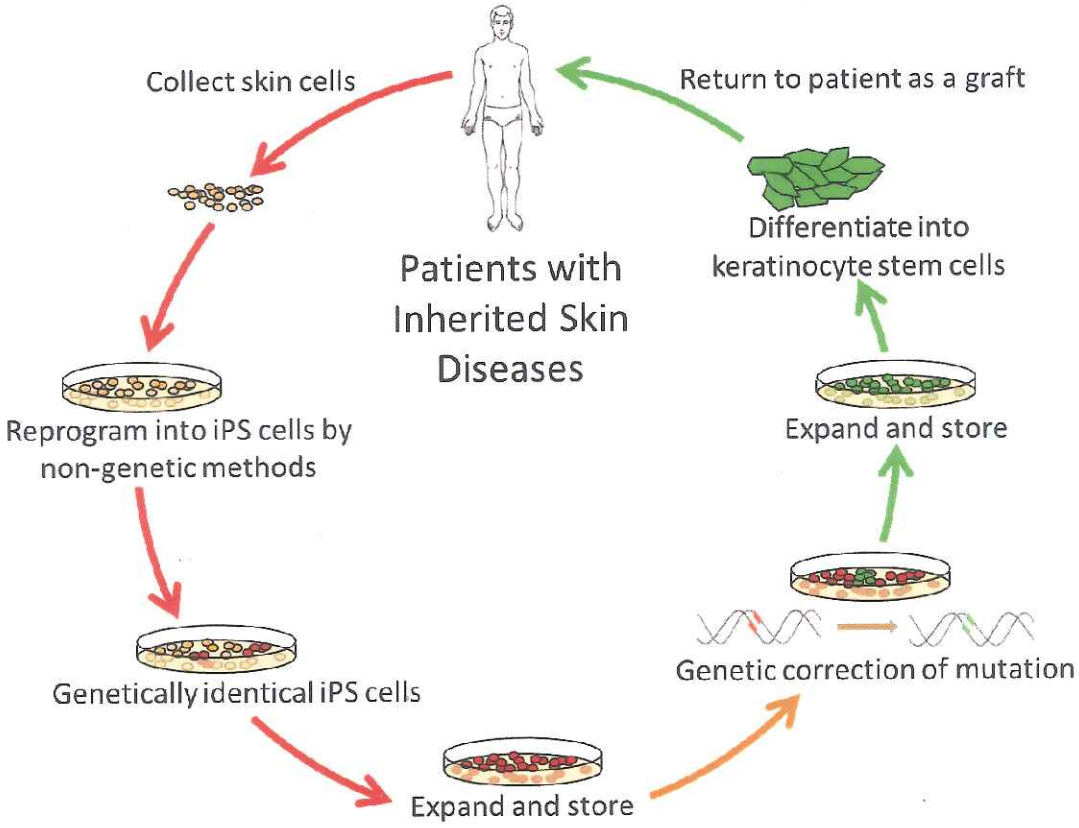
Therapeutic Potential: (iPS) Cells



Therapeutic Potential: (iPS) Cells

- iPS Cell-based Therapies for Parkinson's Disease
- iPS Cell-based Therapies for Cardiovascular Diseases
- iPS Cell-based Therapies for Cartilage and Bone Regeneration
- iPS Cell-based Therapies for Macular Degeneration

Transplanting both a kidney and blood stem cells from the same donor

Preventing organ rejection

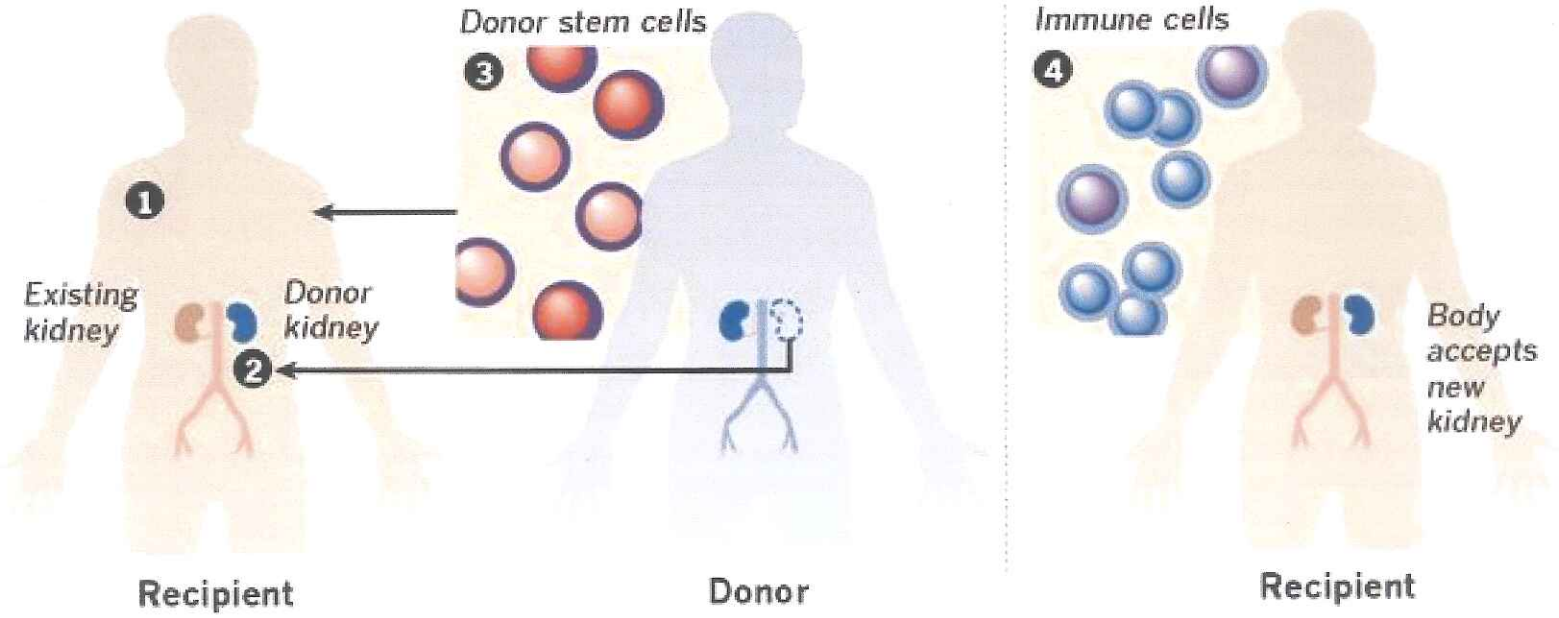
A method that uses the organ donor's stem cells two days after an organ transplant may eliminate an organ recipient's need for lifelong anti-rejection drugs.

❶ Kidney recipient's immune system is suppressed by radiation and chemotherapy.

❷ Donor's incompatible kidney is transplanted into the recipient.

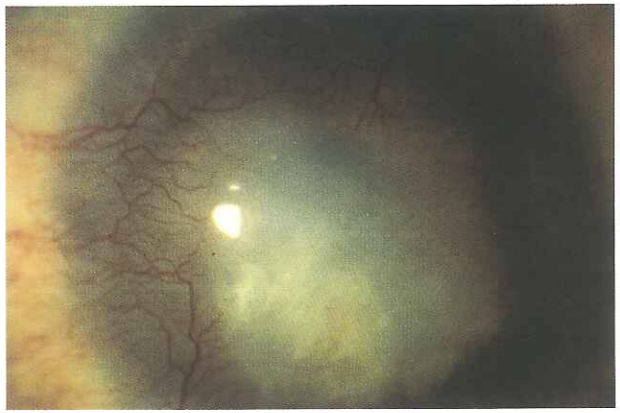
❸ A slurry of stem cells from the donor is transplanted into the recipient.

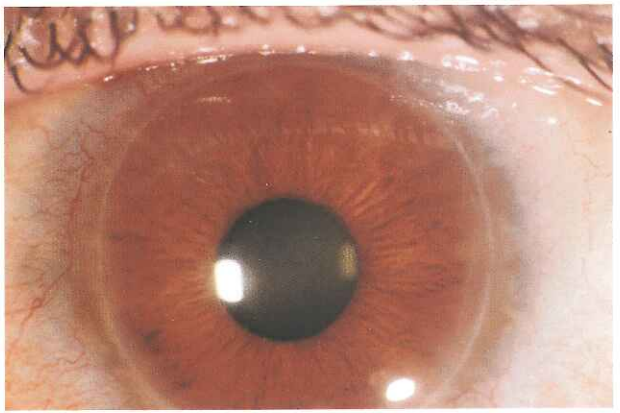
❹ In four weeks, a new immune system has emerged that accepts the new kidney.

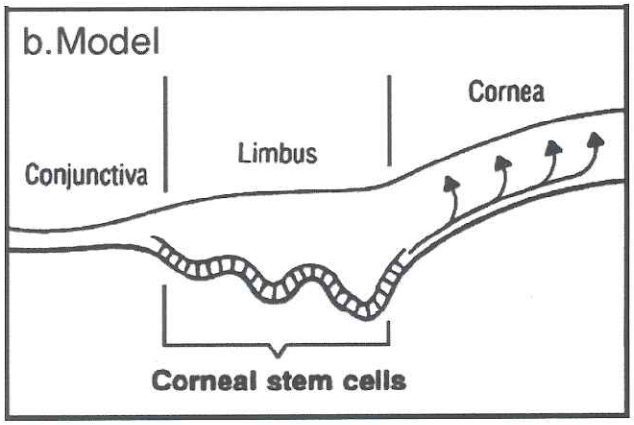


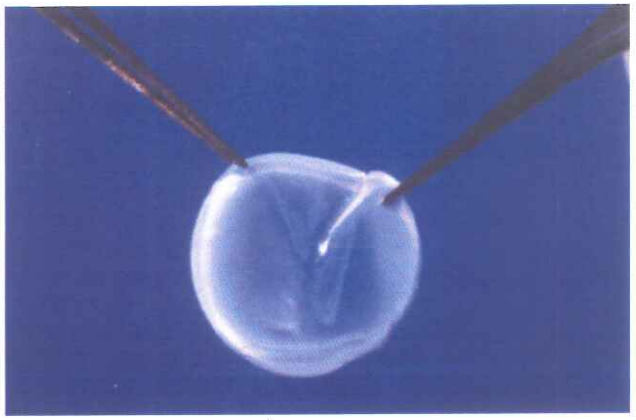
Source: Science Translational Medicine. Graphics reporting by MELISSA HEALY

Restoring Eyesight by Repairing Damaged Corneas using Stem Cells









Courtesy of Dr. Michele De Luca, University of Modena, Italy

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Gates Biomanufacturing Facility

CHANGING THE BIOSCIENCE ECO-SYSTEM IN COLORADO

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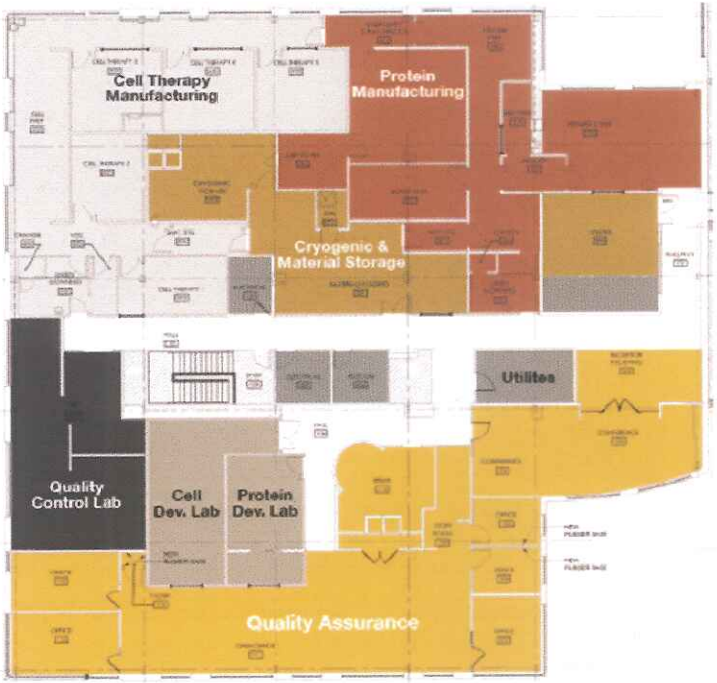
Protein Production and Cell Therapies

Bioscience 1 – 3rd Floor

14,000 ft2 Facility
4,000 ft2 Clean-Room

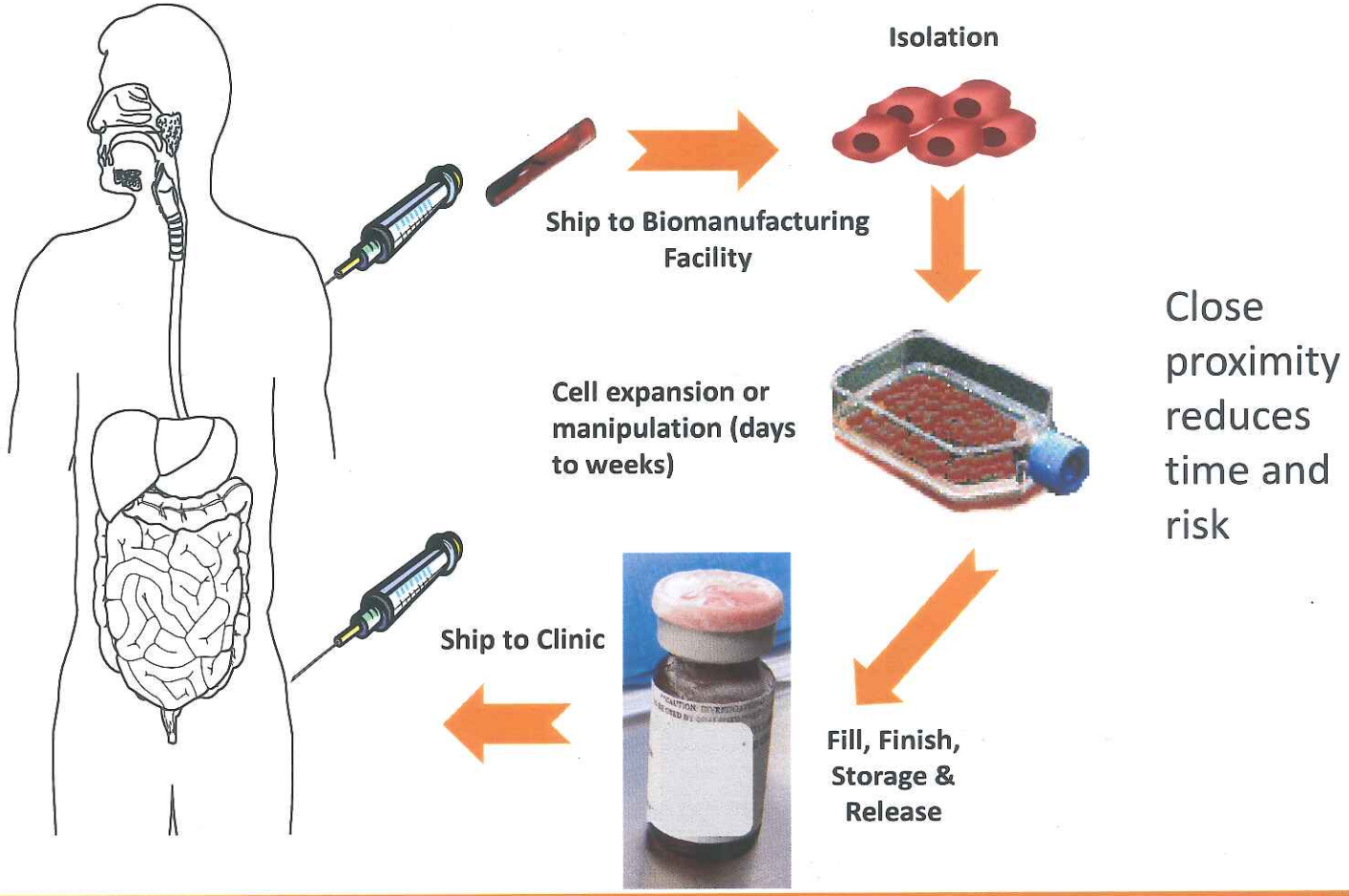
- Cell Therapy Manufacturing (silver)
- Protein Manufacturing (copper)
- Cryogenic & Material Storage (bronze)
- Utilities (pewter)
- Quality Control Lab (black)
- Cell Dev. Lab (nickel)
- Protein Dev. Lab (nickel)
- Quality Assurance (gold)



What is a GMP Facility?

- Complies with FDA's regulations:
  - Controlled manufacturing environment
  - Specified and qualified raw materials
  - Robust operating procedures
  - Reliable testing laboratories
  - Strong quality management systems
- Adherence prevents instances of contamination, mix-ups, deviations, failures, and errors.
- Assures that drug products meet their quality standards.

Patient Interface
Cell therapy products



Close proximity reduces time and risk

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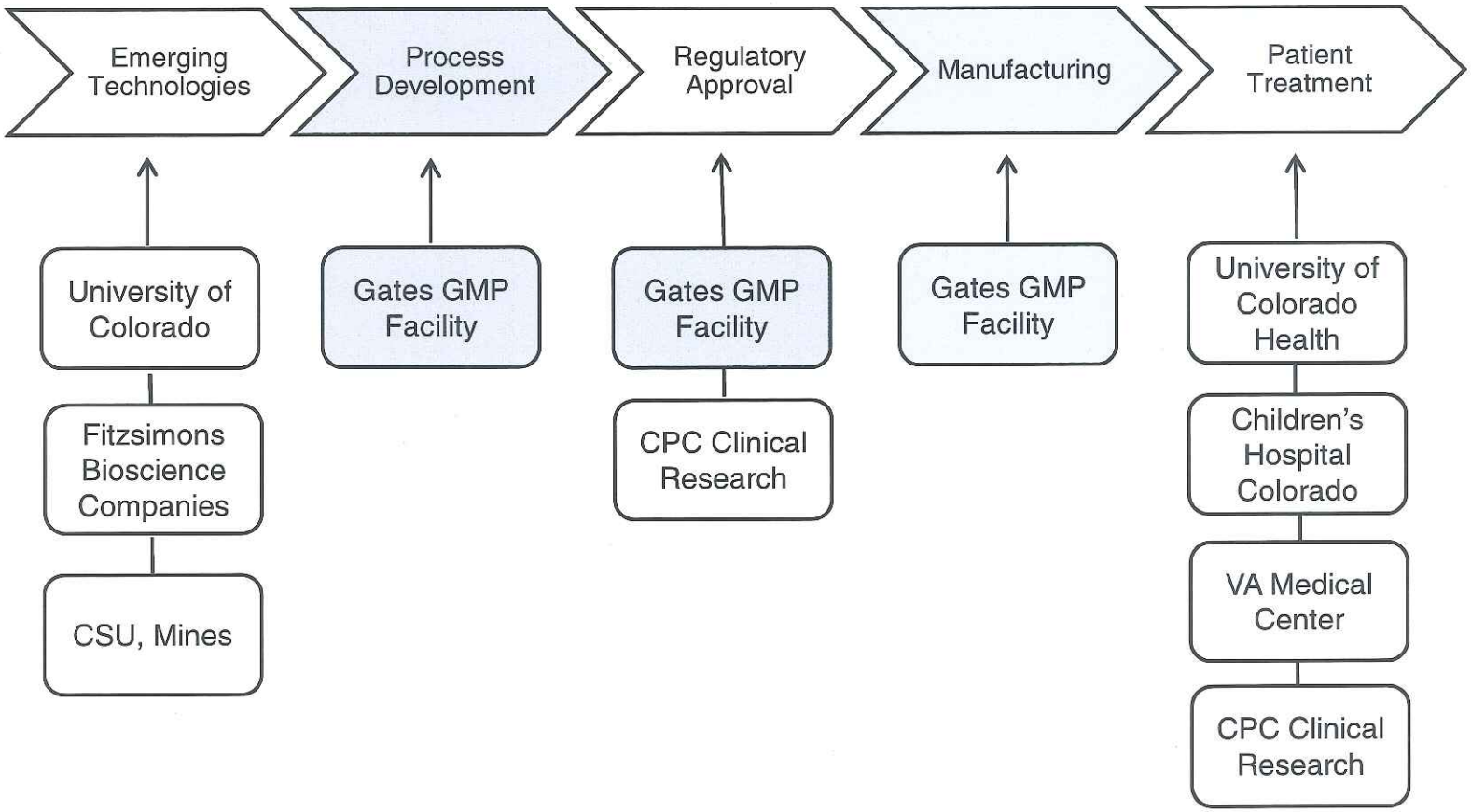
Delivering Regenerative Medicine-Based Technologies throughout Colorado



Charles C. Gates
Biomanufacturing Facility
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Therapies under development

One to Three Years Out

- Corneal Regeneration
- HSC Expansion for immune deficiencies and cancer patients who need BMT
- Oral Mucositis
- Esophageal Repair Following Tumor Removal
- Cell-based Immunotherapies for Treating Cancer

Three to Five Years Out

- Trachea and Wind Pipe Regeneration
- Cell-based Therapies for Cardiovascular Diseases
- Cartilage and Bone Regeneration and Cell-Based Therapies
- Cell-based therapies for macular degeneration
- Cell-based therapies for inherited skin diseases

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24 Academic GMP facilities

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Combined Cell Therapy and Protein Facilities

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Impact

- Recruiting top notch talent
- Clinical Trials-Cutting-edge proteins/biologics and cell-based therapies, including immunotherapies for cancer
- Accelerated Commercialization
- Economic Development
- Improved "Ranking"

Children's Hospital Colorado

UCHealth

School of Medicine
UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

University of Colorado
Anschutz Medical Campus