Protecting the Delivery of Regenerative Medicine or Stem Cell Therapeutics:

Protections Afforded by the Department of Health and Human Services & HRSA's Bureau of Special Programs

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Trustee

New Jersey Stem Cell

Research & Education Foundation

Why is Successful Stem Cell Therapeutic Development Imperative?

- Organ transplant waiting lists continue to grow.
- Number of Americans on chronic mechanical & extracorporeal therapies continues to grow.

- "Baby Boomers" will nearly double the number of Americans over age 65 by 2025.
- Number of Americans without health care insurance may exceed 18% by 2015.

- U.S. health care expenditures on treatment of acute illness, chronic illness & long-term support of individuals in chronically debilitated states may double from $3 to $6 trillion—approaching 40% of U.S. GDP.
- If stem cell parenchymal regeneration enhances quality of life for aging Americans, $3 trillion dollars of U.S. GDP can be redirected.

Current Healthcare Burden by Chronic Debilitating Illness

- Cancer (9 million Americans)
- Heart Failure (5 million Americans)
- Kidney Failure (2 million Americans)
- Liver Disease (4 million Americans)
- Diabetes (16 million Americans)
- Arthritis (25 million Americans)
- Ophthalmologic Disease (12 million Americans)
- Lung Disease (5 million Americans)
- Vascular Disease (13 million Americans)

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91 Million Americans

Current Annual Health Care Expenditures for Chronic Debilitating Illness

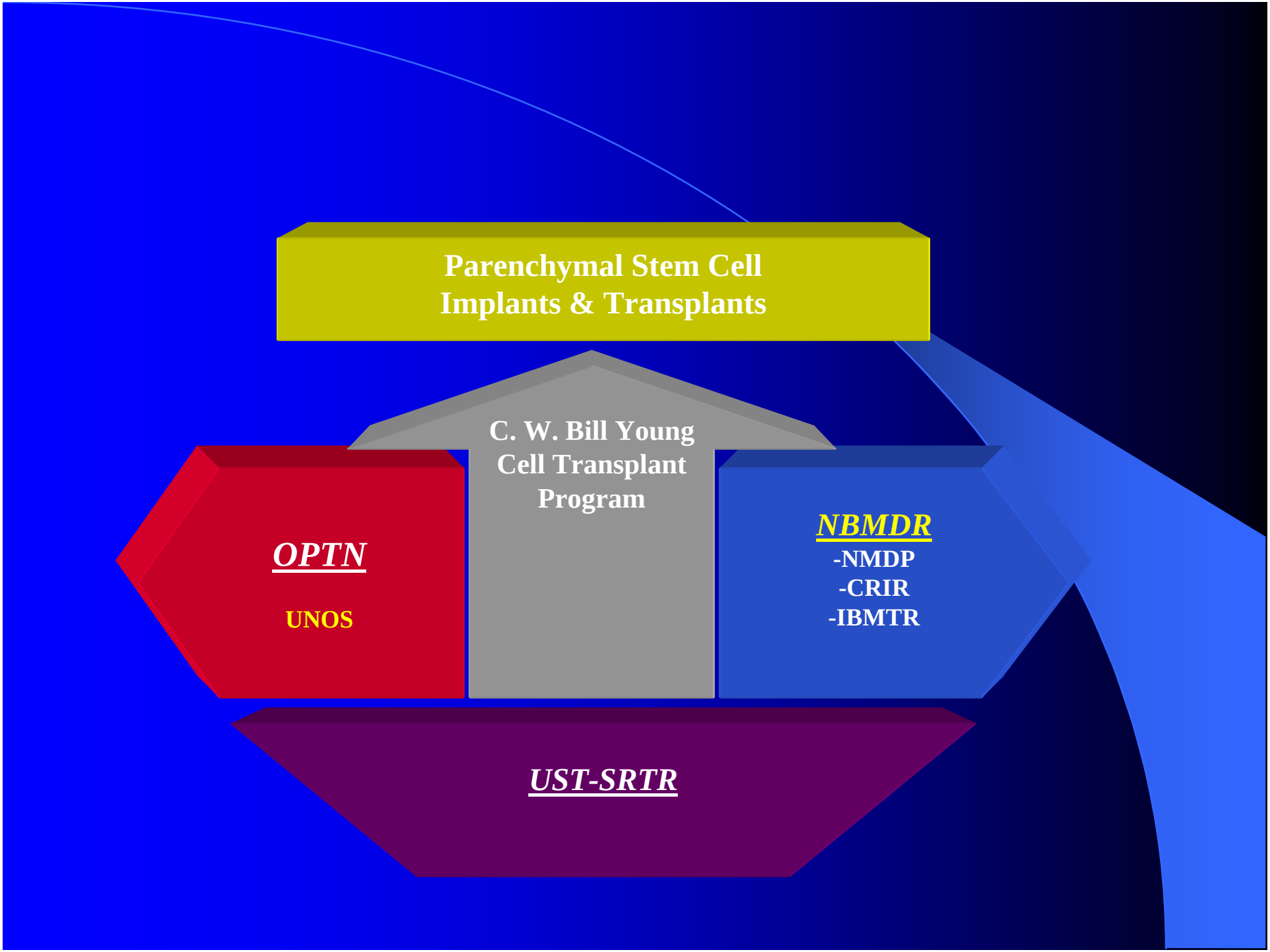
- Cancer
- Heart Failure
- Kidney Failure
- Chronic Lung Disease
- Stroke
- Diabetes
- Arthritis
- Ophthalmologic Disease
- Spinal Cord Injury
- Peripheral Vascular Disease

$3,000,000,000,000.00
Annual Expenditure
(25% of U.S. GDP)

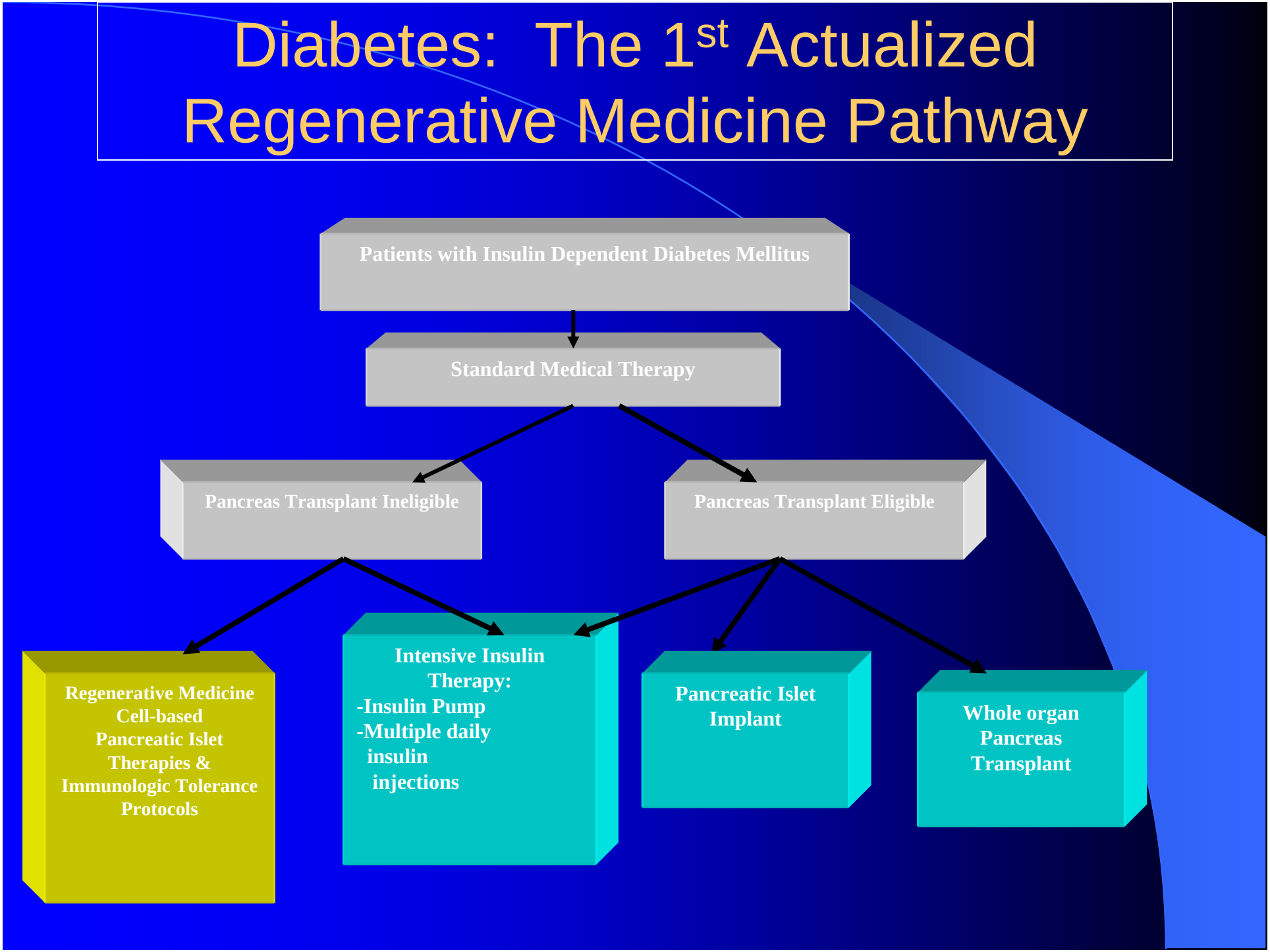
HHS/HRSA Distributes Human Organs/Cells

- 1953—U.S. Organ Transplant begins.
- 1968—U.S. Non-Embryonic Stem Cell Transplants from Bone Marrow commence for cancer treatment.
- 1980—Initial Funding of the National Marrow Donor Program
- 1980s—U.S. Non-Embryonic Stem Cell Transplants expand to include use of Umbilical Cord Blood for cancer treatment.
- 1984—National Organ Transplant Act
  - Sale of human tissue for transplant is federally prohibited.
  - Prevents inequities in organ allocation/distribution by United Network for Organ Sharing.
  - Tissue/Stem Cell Banks are REIMBURSED for supplying transplantable Tissues/Cells
  - Physicians are REIMBURSED for organ procurement & transplant.
- 1997—ESC Patents Established
- 2001—ESC political debate ignites
- 2004—States' Stem Cell funding initiatives
- 2005—C.W. Bill Young Cell Transplant Program (S1317)

NJSCREF
STEM CELL HEALING


Parenchymal Stem Cell
Implants & Transplants
C. W. Bill Young
Cell Transplant
Program
OPTN
UNOS
NBMDR
-NMDP
-CRIR
-IBMTR
UST-SRTR

Diabetes: The 1st Actualized Regenerative Medicine Pathway



Current Pathways for Therapeutic Outlet of Stem Cell Units

Making Patient Safety & Clinical Candor the Hallmark of Regenerative Medicine Clinical Trials: A Proposal for the Pre-emptive Formation of the Stem Cell Injury Compensation Program (SCICP)

- **Establish within HHS/HRSA's Bureau of Special Programs (BSP)—Office of Patient Safety and Health Care Quality and MEDiC Program (Sen. B. Obama & Sen. H. Clinton)**
  - **formalized structure for patients and physicians to have open communication regarding sharing of "up to the minute" data and information—matters that may have far-reaching benefits in regard to the development of Regenerative Medicine.**
  - **Such a program could be the impetus to ensure that "current information" on stem cell lines, which patients might receive in the course of FDA-monitored human clinical stem cell trials, be definable and available to patients and physicians simultaneously**
  - **in keeping with patient advocacy aspects already inherent in the mandates for UNOS and the CW Bill Young Cell Transplant Program**
  - **The existing US-SRTR and any future proposals for patient access to up-to-date information from HHS/HRSA can provide Regenerative Medicine an atmosphere of candor, collaboration and collegiality between potential patients, treating physicians and regulatory bodies**
  - **adoption of a federal stewardship model of FDA/CBER-approved stem cell units by academic, entrepreneurial and other stem cell product developers may facilitate product liability protections afforded by the FTCA. This combination may represent a proposed solution for successful advancement of stem cell therapeutics with far-reaching implications.**

Making Patient Safety & Clinical Candor the Hallmark of Regenerative Medicine Clinical Trials: A Proposal for the Pre-emptive Formation of the Stem Cell Injury Compensation Program (SCICP)

- **Currently stem cell unit risk/benefit modeling can be defined in accordance with the following data points depending upon whether the unit is derived from FDA/CBER-approved sources (non-embryonic) or from not-yet-FDA/CBER-approved sources (embryonic cells):**
  - **existing pre-clinical animal safety/efficacy data with a particular source of stem cells;**
  - **existing pre-clinical animal serious adverse event (SAE) data with a particular source of stem cells;**
  - **existing human clinical safety/efficacy data with a particular source of stem cells;**
  - **and existing human clinical serious adverse event (SAE) data with a particular source of stem cells.**

Medical Malpractice Liability Crisis & Stem Cell Therapeutics

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TRANSPLANTATION

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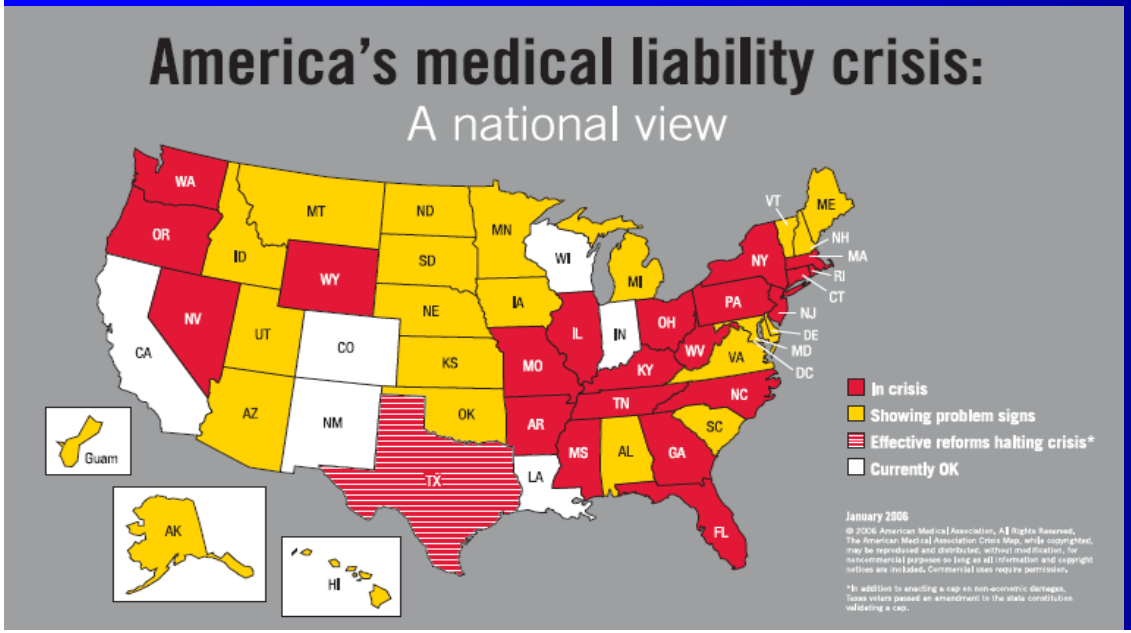
ANALYSES & COMMENTARIES

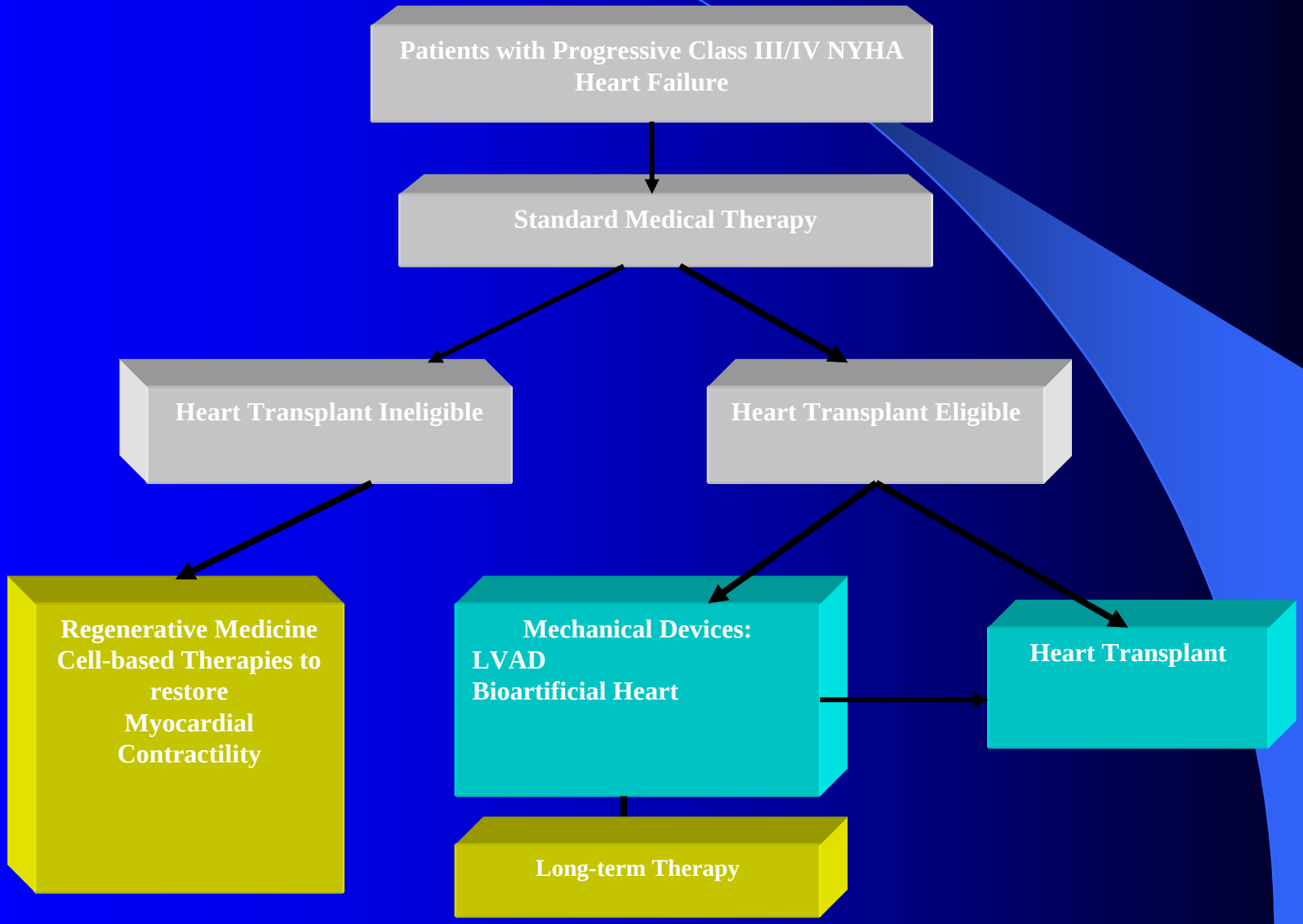
Engraftment and Tumor Formation After Allogeneic In Utero Transplantation of Primate Embryonic Stem Cells. Transplantation 2003; 76: 1061.

T. Asano, N. Ageyama, K. Takeuchi, M. Momoeda, Y. Kitano, K. Sasaki, Y. Ueda, Y. Suzuki, Y. Kondo, R. Torii, M. Hasegawa, S. Ookawara, K. Harii, K. Terao, K. Ozawa, and Y. Hanazono



- **If physicians fear of liability associated with administration of biological (stem cell) therapies is too great, who will administer the fruits of researchers' labor?**

Regenerative Medicine for Chronic Heart Disease



DEVELOPMENT of STEM CELL THERAPEUTICS for U.S. HEALTHCARE DELIVERY:

Proposition for Moving Forward

Proposition for Moving Forward

- **Embrace HRSA Model for Organ and Stem Cell Transplant/Distribution Model;**
  - **Provide the Public easy access to organized animal & human stem cell study data in accordance with UNOS/US-SRTR and NMDP/IBMTR;**
  - **In keeping with NOTA, accept post-transplant reimbursement model for stem cell units (since 1988)**
- **Adopt WHA recommendations to reduce likelihood/risk of human tissue black marketeering;**
- **Incorporate goals of the federally-designated CW Bill Young Cell Transplant Program National Stem Cell Repository;**
- **Consider recently proposed Office of Patient Safety & Health Care Quality and Medical Error Disclosure & Compensation Program (MEDiC Program)**
- **Adopt the FTCA and Project Bioshield protection measures to ensure avoidance of excessive product liability and the stifling of Regenerative Medicine.**
  - **Preemptively establish a Stem Cell Injury Compensation Program Fund that incorporates risk factors specific to each class of stem cells;**

ClinicalTrials.gov

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