Midwest Stem Cell Therapy Center

Status Update
March 7, 2017

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Director, Division of Cardiovascular Diseases
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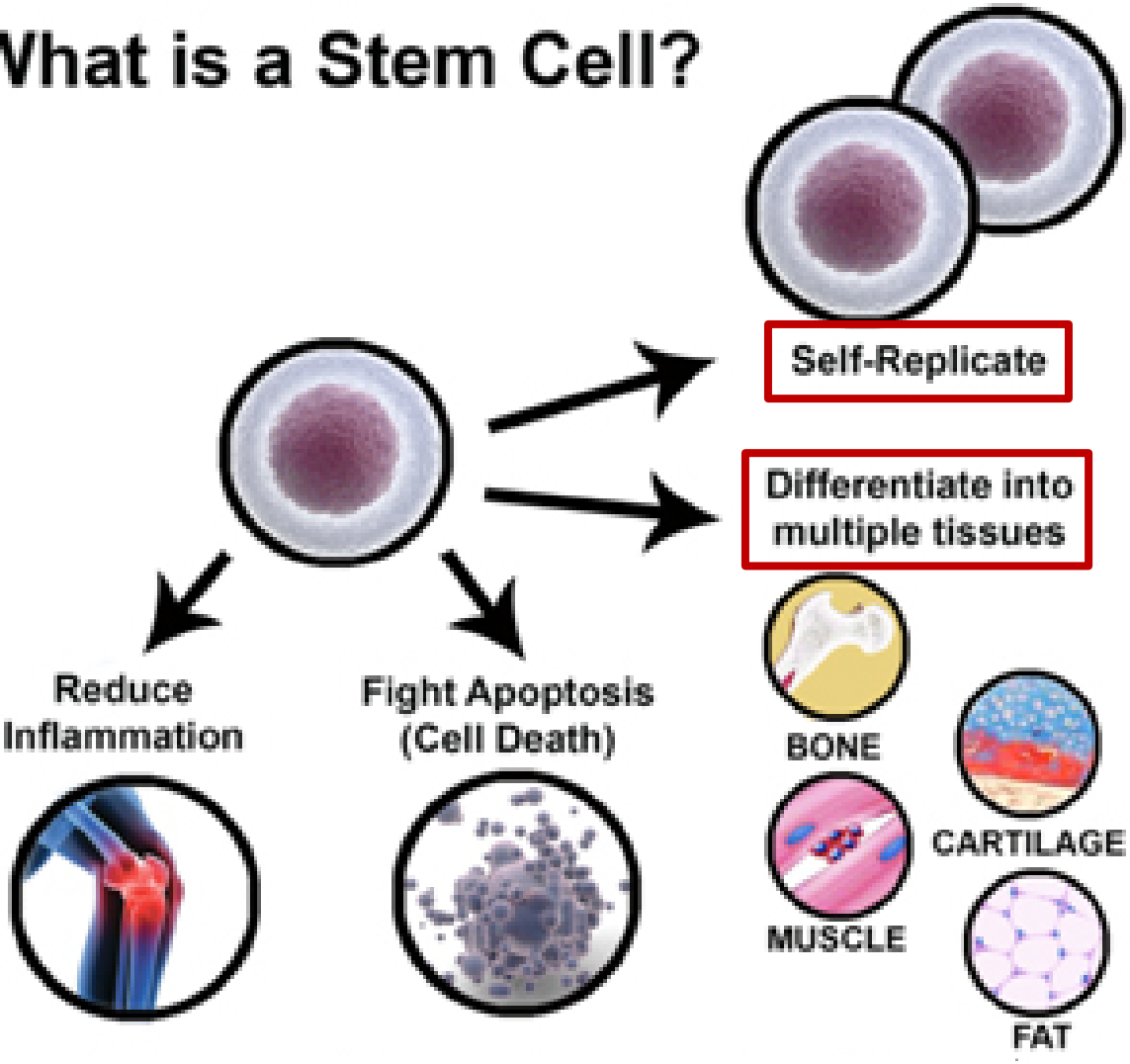
KU MIDWEST STEM CELL THERAPY CENTER
The University of Kansas

THE UNIVERSITY OF KANSAS HOSPITAL

What is a Stem Cell?



Heart Repair with Adult Bone Marrow Cells

letters to nature

Bone marrow cells regenerate infarcted myocardium

Donald Orlic†, Jan Kajstura*, Stefano Chimenti*, Igor Jakoniuk*, Stacie M. Anderson†, Baosheng Li*, James Pickel‡, Ronald McKay‡, Bernardo Nadal-Ginard*, David M. Bodine†, Annarosa Leri* & Piero Anversa*

** Department of Medicine, New York Medical College, Valhalla, New York 10595, USA*
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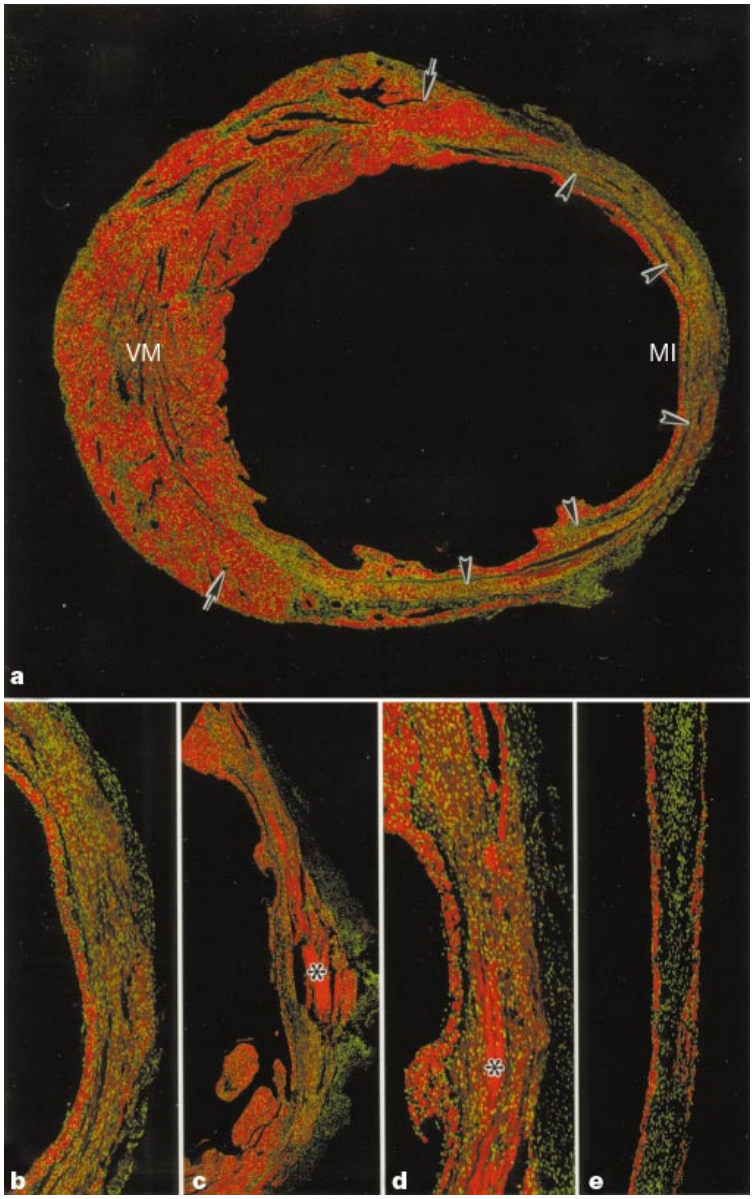
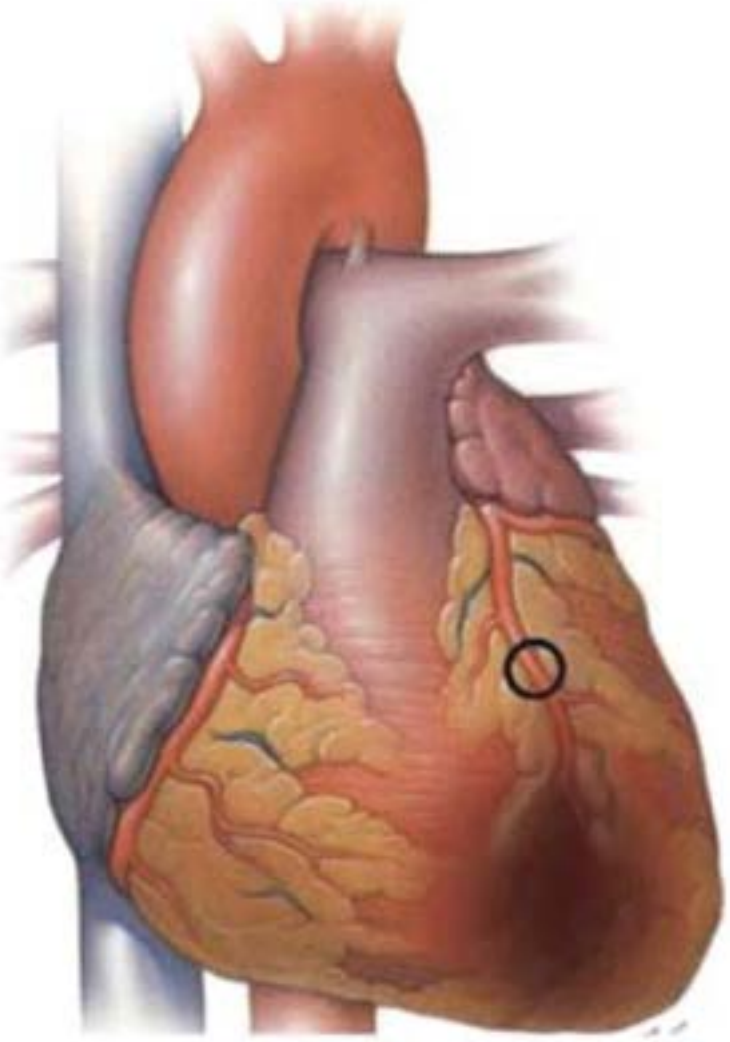
NATURE | VOL 410 | 5 APRIL 2001 | www.nature.com

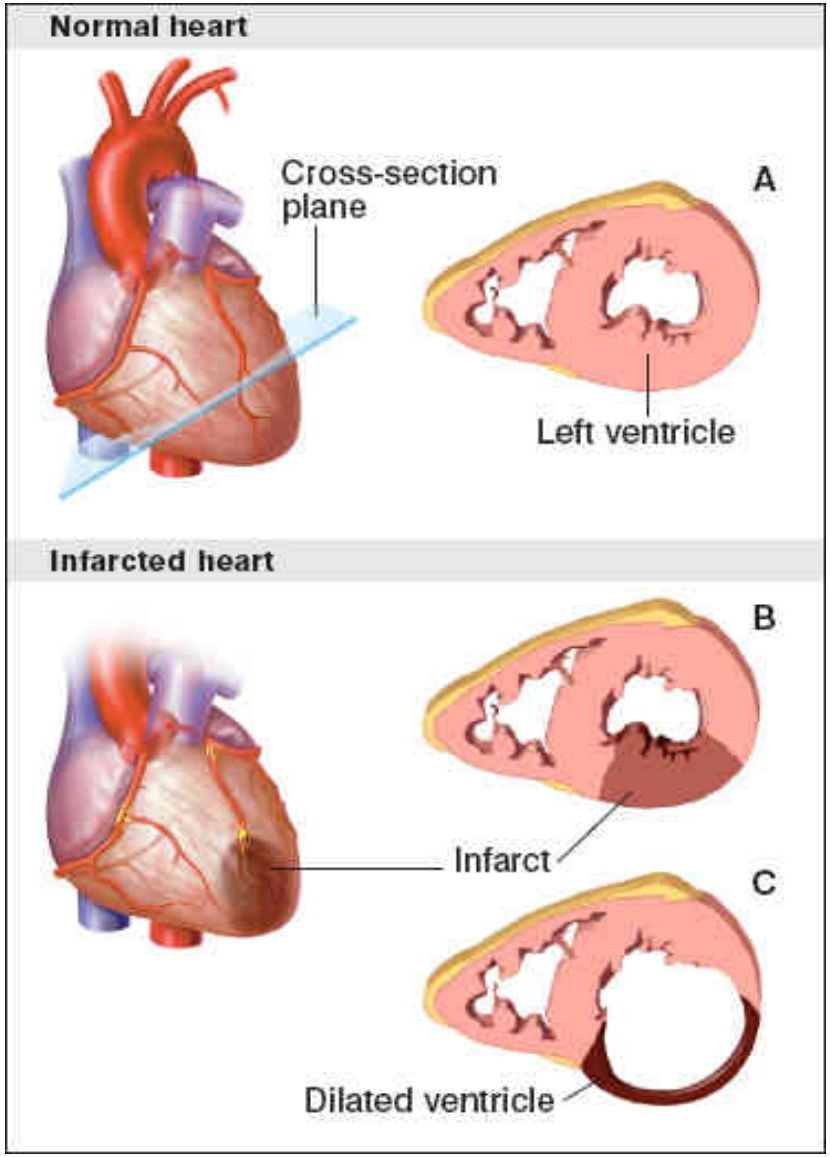


Nature. 2001 Apr 5;410(6829):701-5.

Lin-/c-kit+ BMCs

Infarcted Heart – Target for Adult Stem Cell Therapy



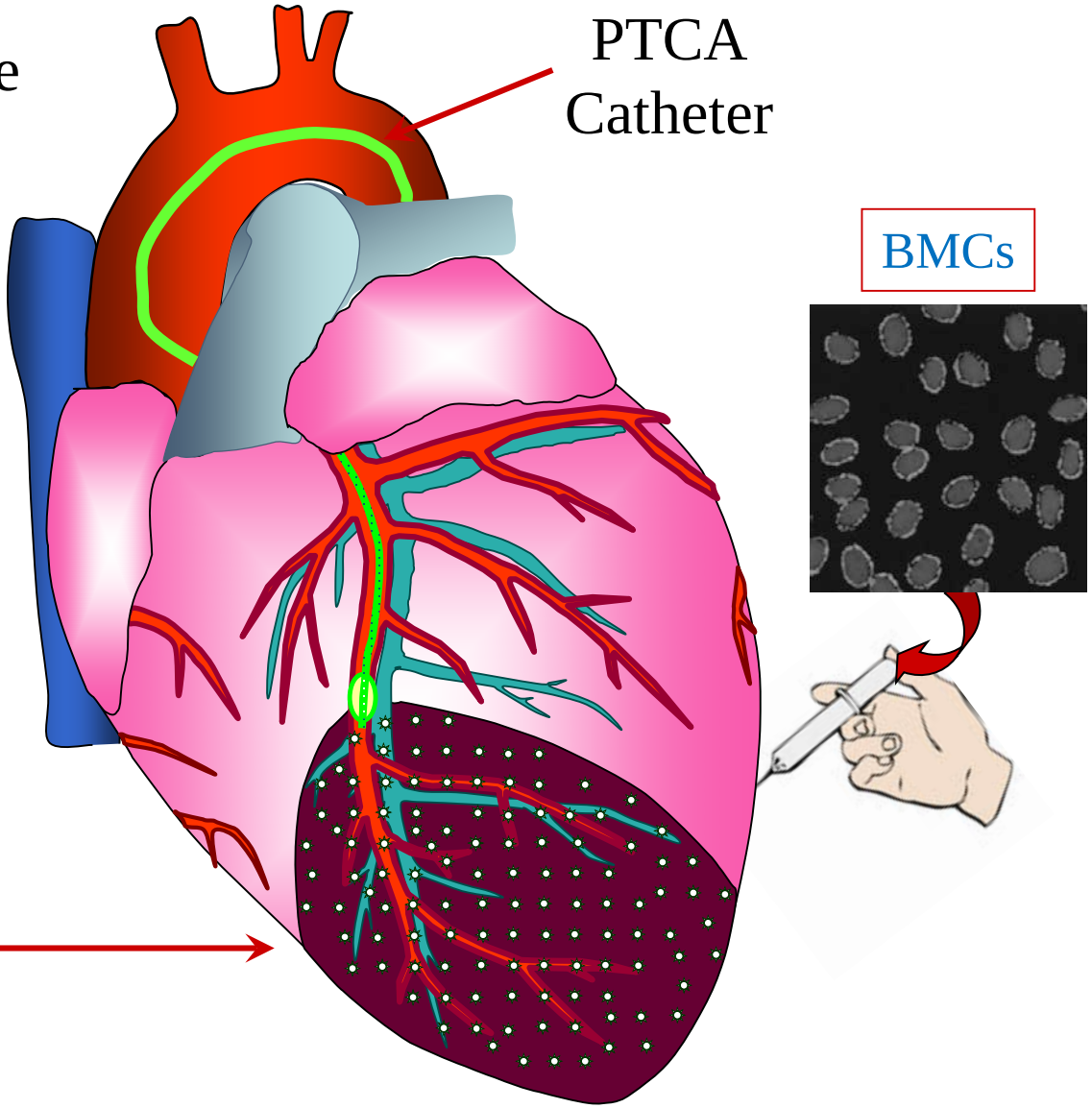


Thygesen *et al.* J Am Coll Cardiol 2012;60:1581-98.

Source: http://www.health-pic.com/complications-of-myocardial-infarction/

Injection of BMCs for Heart Repair in Humans

More than 60 studies have been completed and published



Damaged area with BMC infusion

Adult Stem Cells for Heart Repair

- Bone marrow mononuclear cells
- BM Mesenchymal stem cells (MSCs)
- Circulating progenitor cells (CPCs)

Numerous clinical trials - completed and ongoing

- Cardiac stem cells — 2 clinical trials
- Adipose stem cells — 2 clinical trials
- Skeletal myoblasts — Several clinical trials completed
- Cord blood cells

Afzal et al. *Circulation Research* 2015;117:558-575

Adult Bone Marrow Cell Therapy for Ischemic Heart Disease

Evidence and Insights From Randomized Controlled Trials

Muhammad R. Afzal, Anweshan Samanta, Zubair I. Shah, Vinodh Jeevanantham, Ahmed Abdel-Latif, Ewa K. Zuba-Surma, Buddhadeb Dawn

From the Division of Cardiovascular Diseases, Cardiovascular Research Institute, and the Midwest Stem Cell Therapy Center, University of Kansas Medical Center, Kansas City (M.R.A., A.S., Z.I.S., B.D.); Heart and Vascular Specialists of Oklahoma, Oklahoma City (V.J.); Division of Cardiology, University of Kentucky, Lexington (A.A.-L.); and Department of Cell Biology, Faculty of Biochemistry, Biophysics and Biotechnology, Jagiellonian University, Krakow, Poland (E.K.Z.-S).

- 48 RCTs were included in this meta-analysis
- A total of 2,602 patients
  (1,468 BMC-treated and 1,134 control patients)

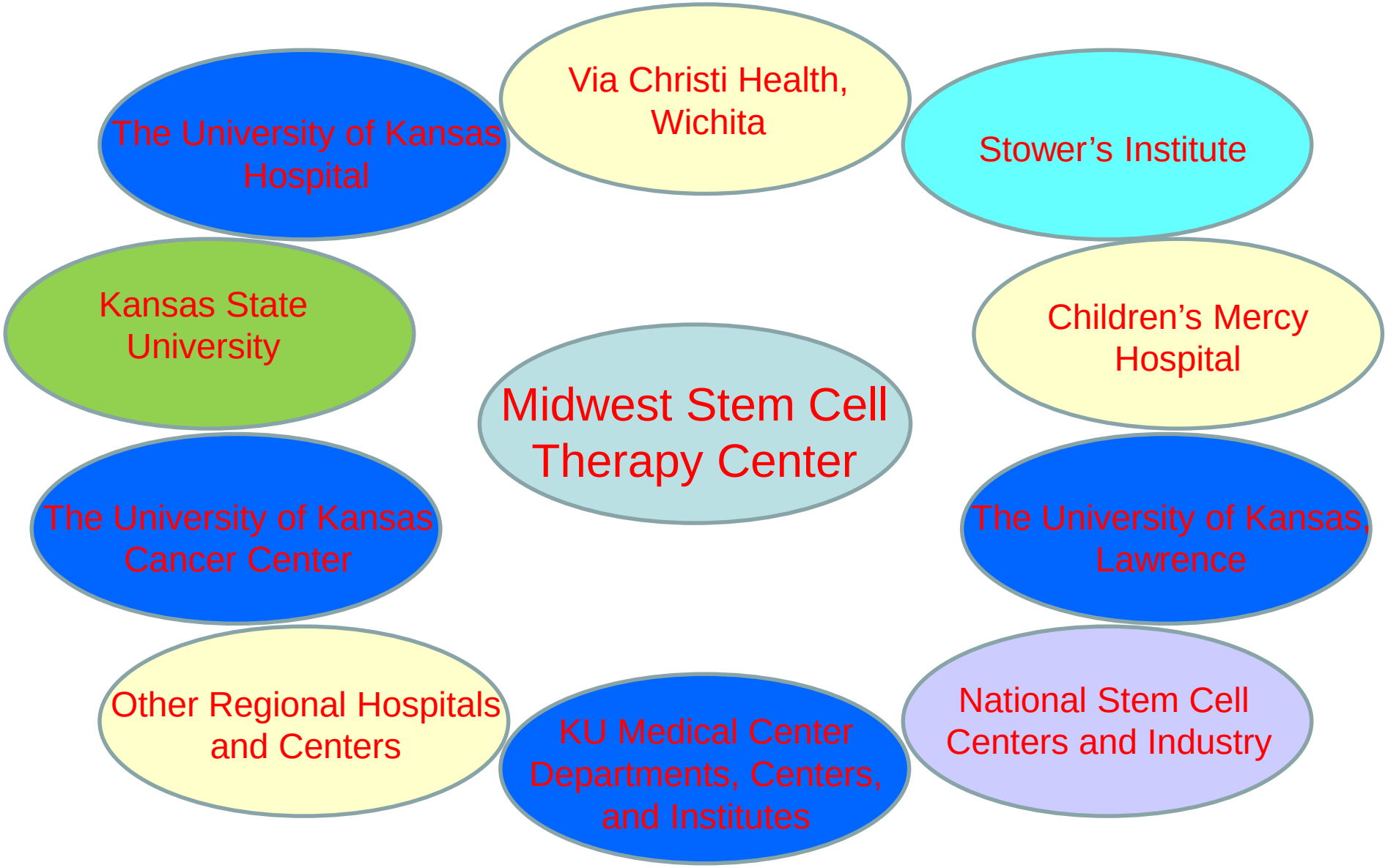
Compared with controls, in BMC-treated patients:

- LVEF improved by 2.92%
- LV end-systolic volume decreased by 6.37 ml
- LV infarct scar size reduced by 2.25%
- LV end-diastolic volume decreased by 2.26 ml (P=0.06)

NHLBI Centers for Stem Cell Therapy

Senate Bill 199

Stem Cell Hub



Objectives of the Center - I

- To advance adult, cord blood and related stem cell research and therapies for patient treatment
- To serve as a core facility to produce clinical grade stem cells
- To initiate clinical trials with adult, cord blood, and related stem cells

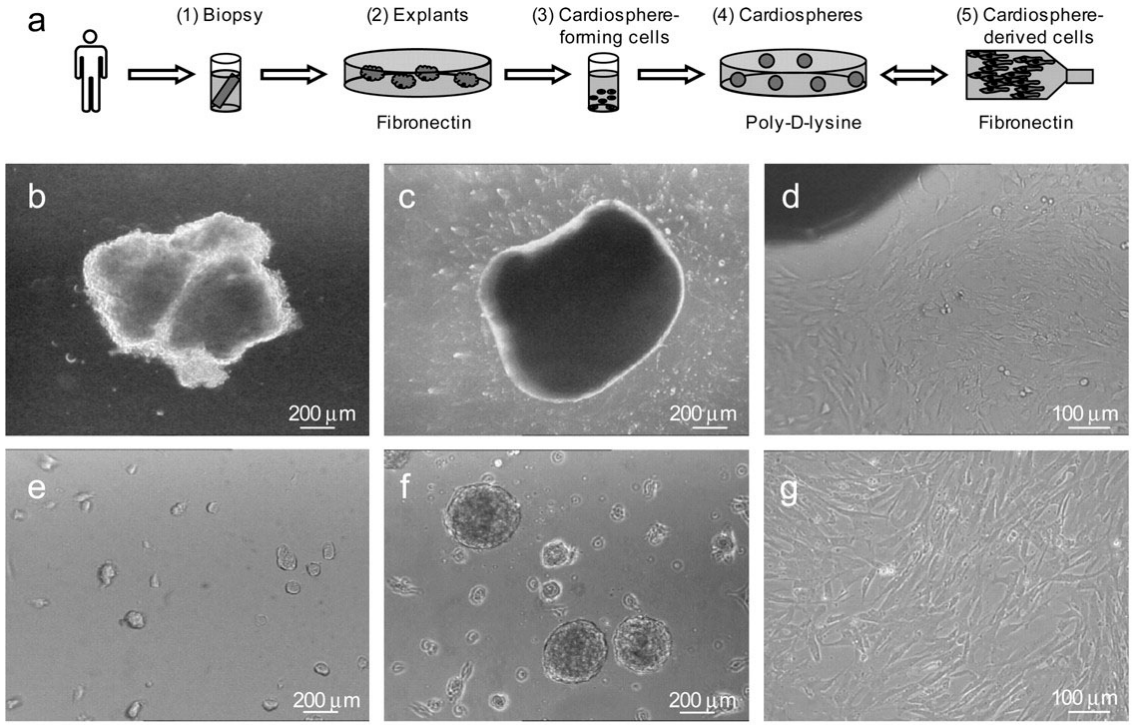
Good Manufacturing Practice Facility

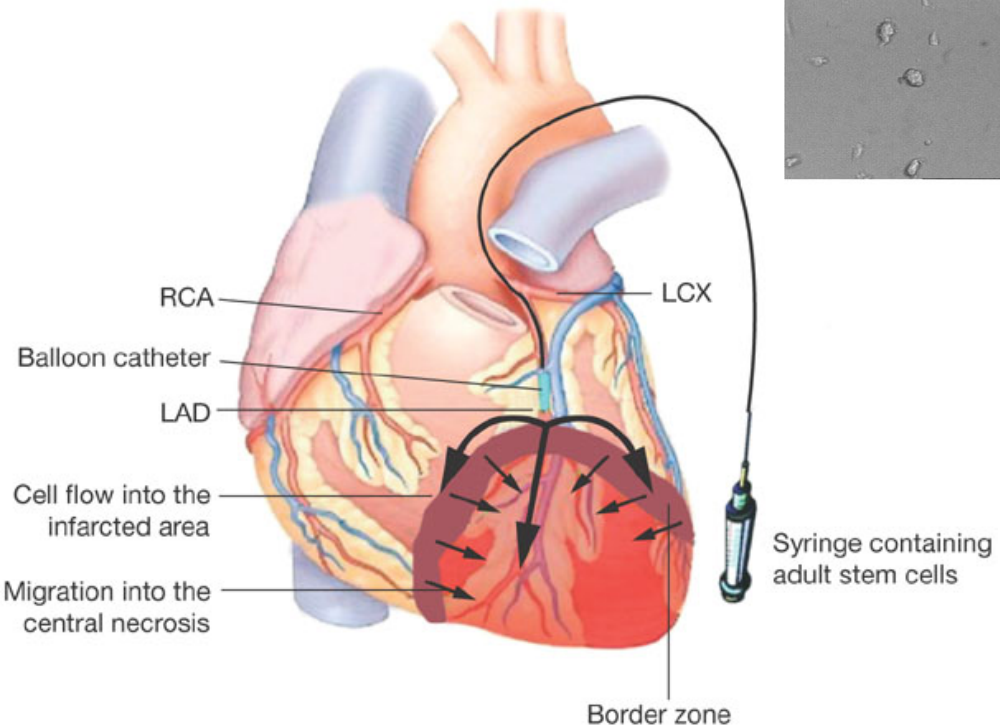
Focus Areas for Adult Stem Cell Therapy

- Stroke and Neurodegenerative diseases
- Cancer and immunotherapy
- Cardiac and vascular
- Musculoskeletal, trauma, skin, burn, wounds, autoimmune diseases

Clinical Trials for Heart

ALLSTAR:
Heart-derived stem cells for patients with heart attack





PreSERVE AMI:
Bone marrow-derived CD34+ cells for patients with heart attack

Clinical Trial for Stroke

ACTIsSIMA

- Modified adult bone marrow stem cells (SB623) for patients with motor deficiency following an ischemic stroke
- US Biotech company sponsoring trial
- Open to enrollment

Clinical Trial for Cancer

- Graft vs. Host Disease
  - Wharton's Jelly mesenchymal stem cells to be injected intravenously to combat GvHD
  - Methods to expand cells under GMP conditions have been developed
  - Preclinical studies near completion
  - IND to be submitted to the FDA soon

Preclinical Projects

Amyotrophic Lateral Sclerosis (Lou Gehrig's Disease)

- KU Dept of Neurology collaboration
- Initial Proof of Concept studies ongoing

Liver repair

- KU Department of Pharmacology and Toxicology collaboration
- Pre-PreIND items being developed

Additional KUMC Collaborations

- Heart scar repair
  - KUMC, Cardiovascular Research Institute
- Spinal cord repair
  - KUMC, Dept. of Molecular and Integrative Physiology
- Stroke and traumatic brain injury
  - KUMC, Dept. of Rehabilitation Medicine
- Cartilage Repair
  - KUMC, Dept. of Orthopedic Surgery

Objectives of the Center - II

- Informing the public on available adult, cord blood, and related stem cell therapeutic options
- Creating and maintaining a database of available stem cell clinical trials and therapies
- Foster a regional network of physicians trained in adult stem cell therapy

Dissemination of Information

- Website (www.kumc.edu/msctc)
- Compilation of an extensive resource for adult stem cell information
- Providing answers via emails/meetings
- Conferences

Midwest Conference on Cell Therapy and Regenerative Medicine

September 16-17, 2016
Sheraton Overland Park, KS

MSCTC Fiscal Overview

The MSCTC is currently funded by:

- State of Kansas annual appropriation
- Donor giving routed through KU Endowment
- EVC discretionary KUEA fund for Advisory Board and other official hospitality expenses
  - “Official hospitality” needs to be added to the State budget bill
- Income received from GMP manufacturing and externally funded projects
- Clinical Trials

Major Support from KU Medical Center and the Kansas Legislature

- Funds toward initial GMP construction, personnel salary and benefits
- Space and other key infrastructure support
- Administrative support
- Brand recognition
- Abundance of collaborators
- Continued funding from the State of Kansas

Business Initiatives

Sponsored R&D and Fee-for-Service

- Dental pulp MSC company
  - Isolation, recovery of dental pulp MSCs and long-term banking
  - Contract for business over the next 20 years executed
- Gene therapy company
  - Gene Therapy for Aplastic Anemia
  - Contract in development
- Company operating clinics
  - Adipose MSC treatment for Osteoarthritis/Cartilage Repair
  - Project discussions to resume this week
- Cord blood and tissue storage company
  - Fee for service for developing methods, processing blood and tissue, and long-term storage
  - Project scope and budget discussion underway

Clinical Trials

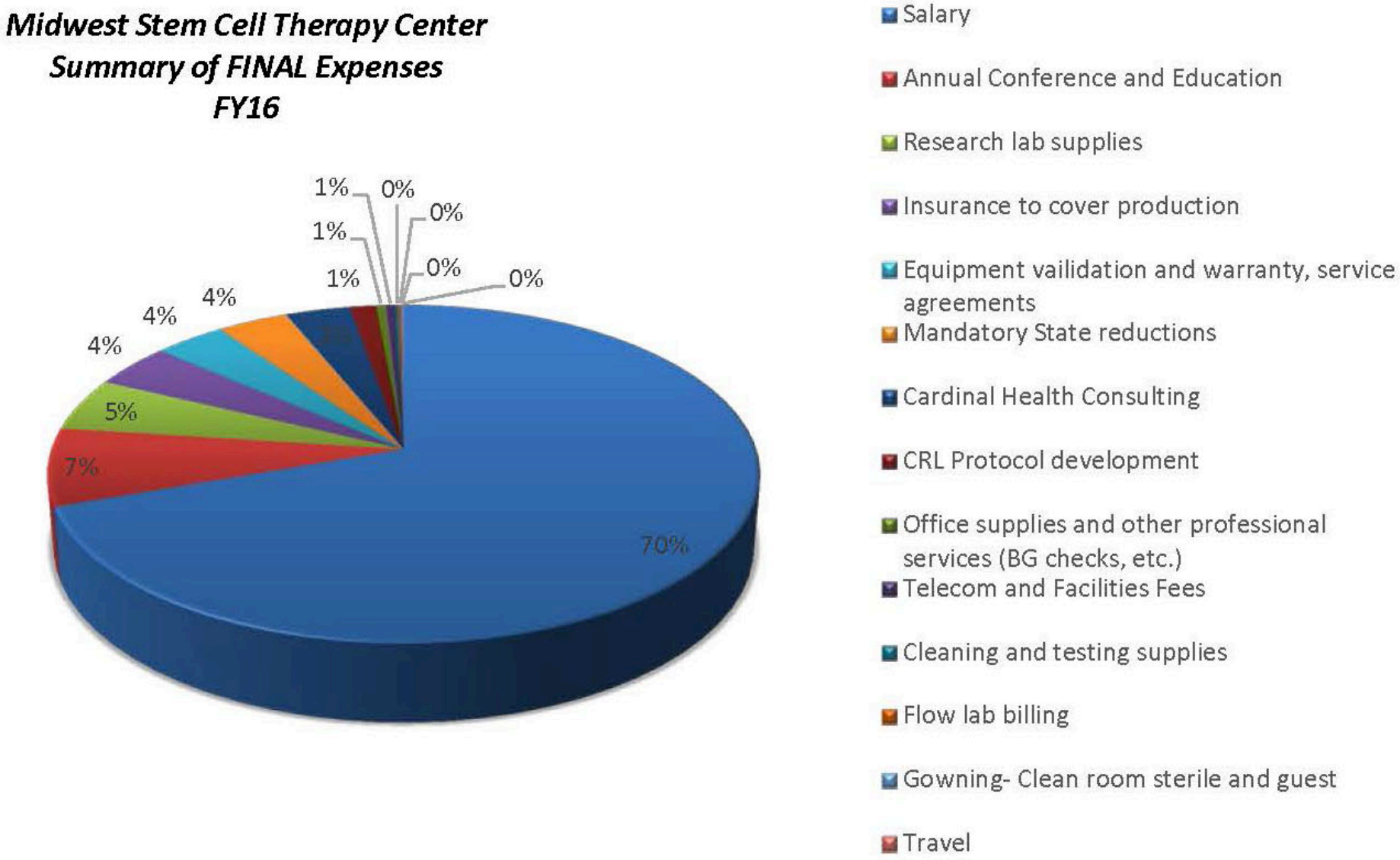
FY16 Expense Report

State appropriations

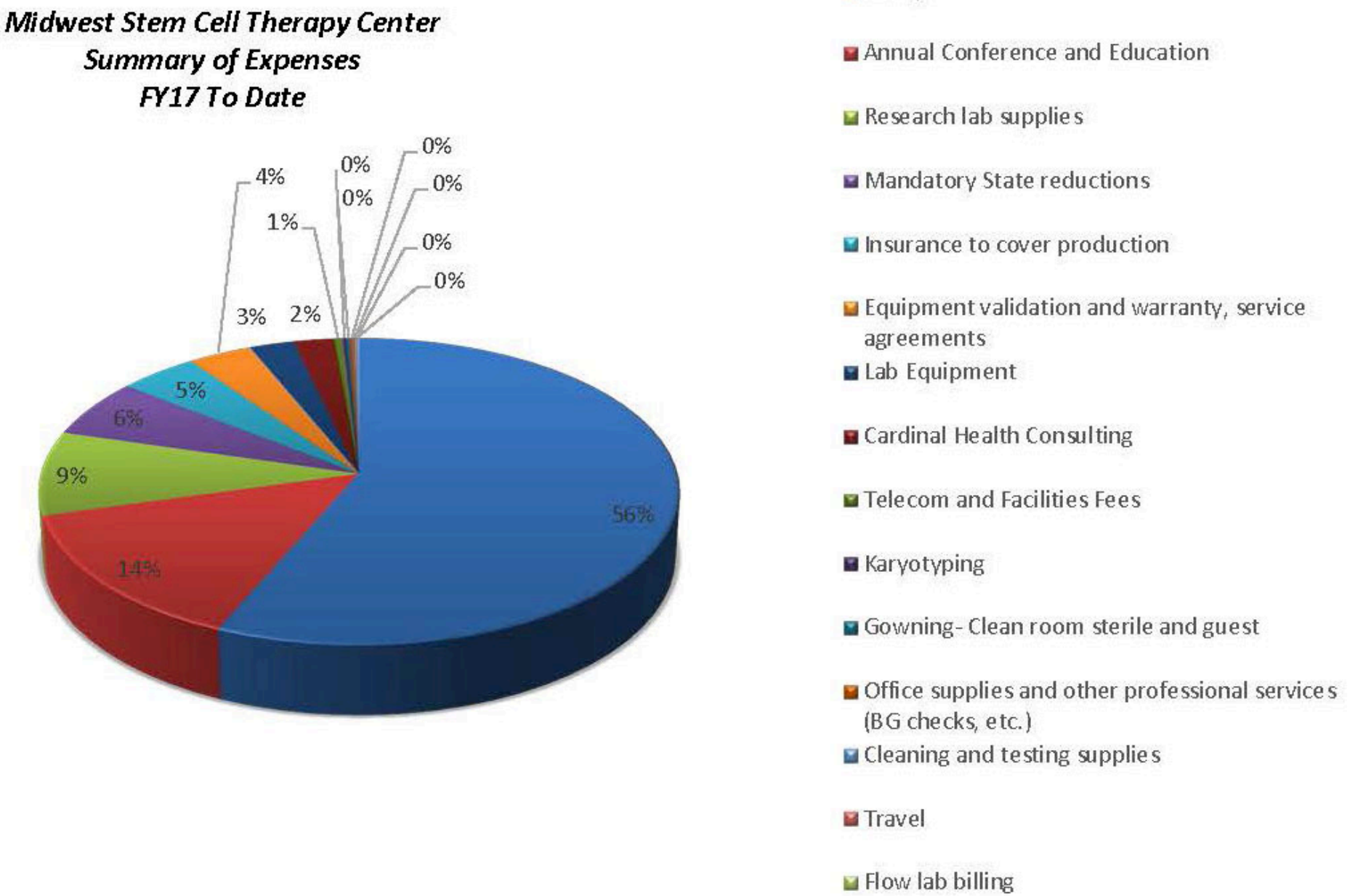
FY16 – Total amount received: $754,500

Expenses		% of FY total
Salary	$525,117.65	69.60%
Annual Conference and Education	$56,300.00	7.46%
Research lab supplies	$38,251.00	5.07%
Insurance to cover production	$31,800.00	4.21%
Equipment validation and warranty, service agreements	$28,449.49	3.77%
Mandatory State reductions	$27,879.00	3.70%
Cardinal Health Consulting	$25,873.07	3.43%
CRL Protocol development	$10,000.00	1.33%
Office supplies and other professional services (BG checks, etc.)	$3,875.17	0.51%
Telecom and Facilities Fees	$3,632.82	0.48%
Cleaning and testing supplies	$1,524.24	0.20%
Flow lab billing	$1,101.57	0.15%
Gowning- Clean room sterile and guest	$576.99	0.08%
Travel	$119.00	0.02%
FY16 Final Expenses Total	$754,500.00	

FY16 Expense report



FY17 Sources and Spends (Year to Date)



Midwest Stem Cell Therapy Center: Accomplishments

- Brought industry-sponsored adult stem cell therapy trials to Kansas and Midwest
- Built a GMP, started the production of clinical grade adult stem cells
- Close to initiating home-grown stem cell therapy trial for cancer patients
- Initiated business and fee-for-service collaborations, generating revenue
- Collaborating widely both within and outside of KU with regional institutions as well as businesses
- Trained students and researchers
- Conducted 4 very successful adult stem cell conferences
- Created a database for adult stem cell information on the web
- Established MSCTC presence on the web
- Addressing patient concerns and comments on a regular basis, providing information about adult stem cells
- Performing cutting-edge basic science research with iPSC and other cells

MSCTC Vision for Growth

- Advance cutting-edge adult stem cell therapy
    - Multi-center adult stem cell trials
    - Deliver therapy locally in Kansas in the form of trials through clinics
    - Promote local basic/clinical investigators

- Produce patient-specific adult stem cells locally and generate new business
    - Expand the physical capacity/facility

- Advance basic stem cell research
    - Innovation

Strategic Plan Development

Center Profile

- Grounding in KUMC's strategic plan
- Set center's mission and vision
- SWOT analysis
- Program, service and customer requirements

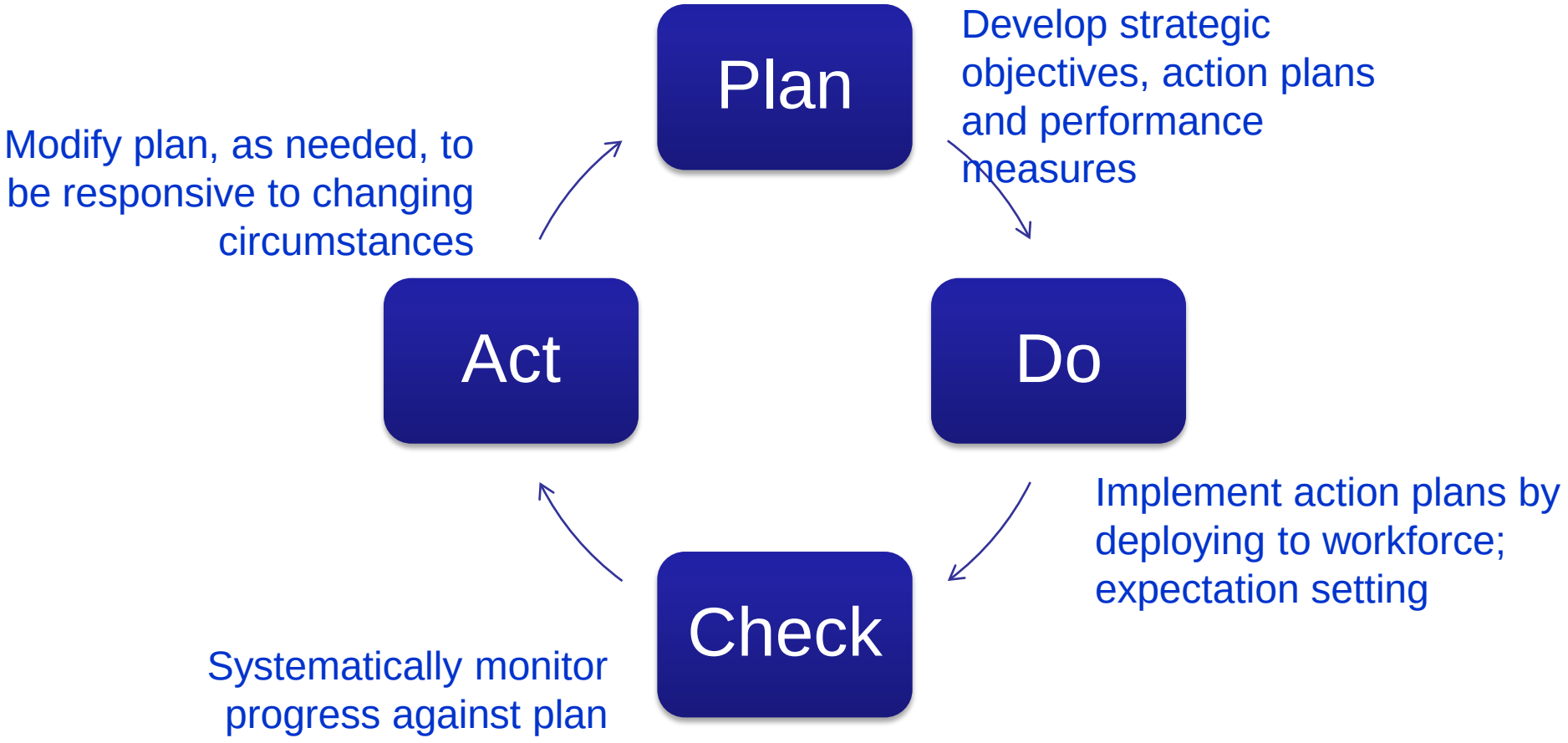
Goal Setting

- Identify potential goals that support the mission and vision
- Prioritize goals
- Develop action plans

Implementation

- Execute on action plans
- Monitor plan progress
- Modify and improve plans, as needed

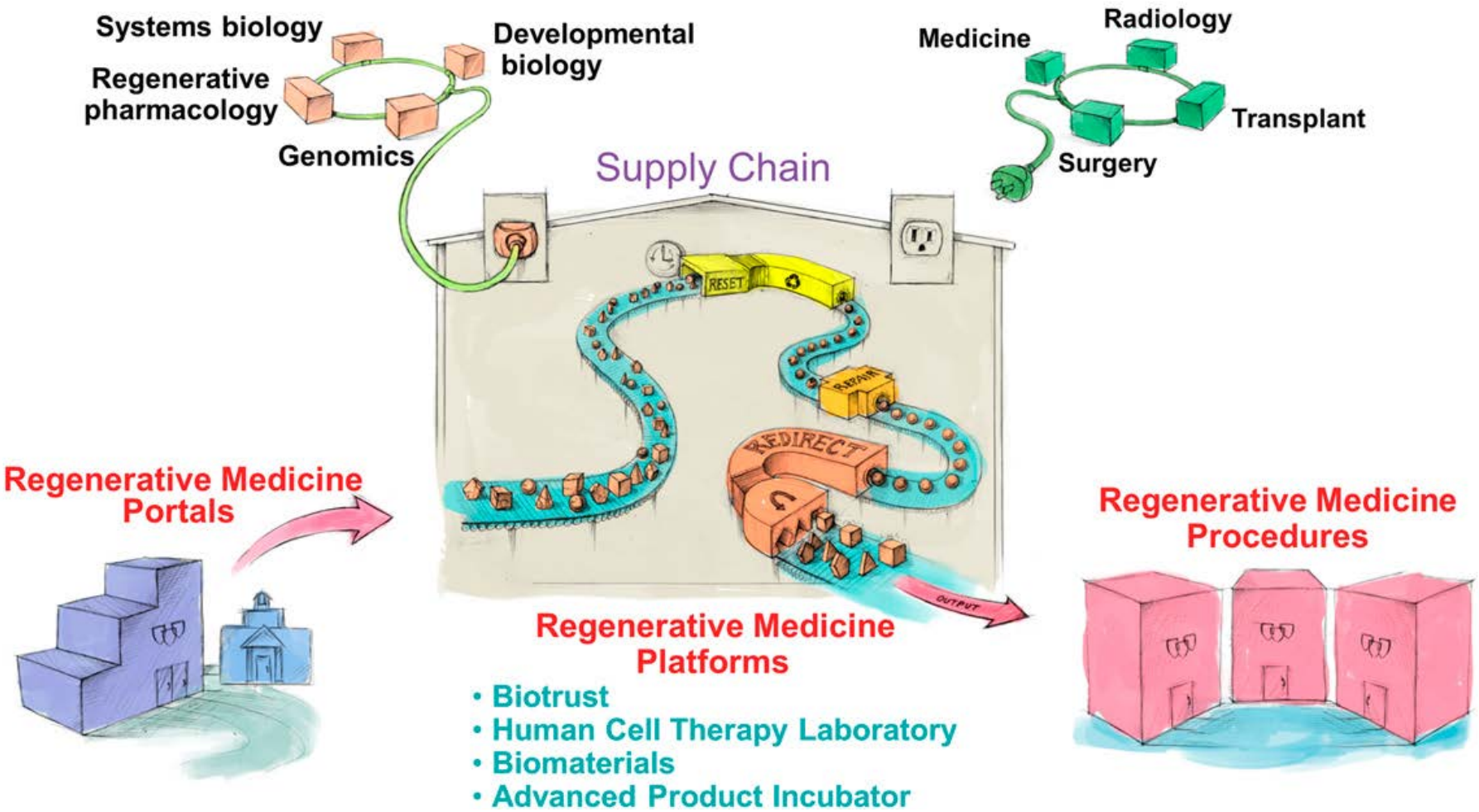
Strategic Plan Deployment



MSCTC 2.0

Terzic et al. *Stem Cells Translational Medicine* 2015;4:1-7

Regenerative Medicine Service Line



Conclusions

- Adult stem cell therapy can potentially cure diseases that are major health problems
- The importance of a local adult stem cell center delivering adult stem cell therapy to Kansans cannot be overemphasized
- MSCTC has been successful within its means
- A strategic planning will take this highly functional center to the next level to deliver adult stem cell therapies and potentially benefit many more patients in a large territory

THANK YOU