



Sustainable outcomes in
stem cell and genomics
research - the role
of responsible
leadership

Michael S. Pepper
MBChB, PhD, MD



UNIVERSITEIT VAN PRETORIA
UNIVERSITY OF PRETORIA

Institute for Cellular and Molecular Medicine

Institute for Cellular and Molecular Medicine

- Initiated in August 2008
- 2011 - Faculty Research Theme
 - 2012 - Funding from UP Executive
- 20 groups at present
- 7 Faculties
 - Health Sciences, Natural and Agricultural Sciences, Veterinary Sciences, Law, Economic and Management Sciences, EBIT, Humanities

Thematic areas

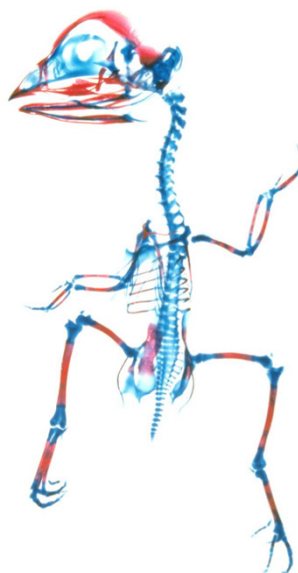
- Stem cell research and therapy
- Genetics/genomics
- Neurosciences (incl. neuroendocrinology)
- Inflammation and immunity
- Cross-cutting
 - Human tissue legislation
 - Bioentrepreneurship

Cells of the human body

- > 200 different cell types in the human body
- Adult human made up of $\sim 10^{13-14}$ cells
 - Human body also contains $\sim 10^{13}$ microbes
- ~ 200 million (2×10^8) cells lost from the body of an adult per minute

Adult stem cells

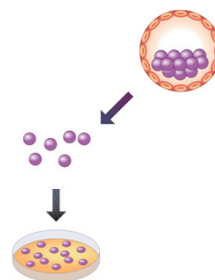
- bone marrow
- gastrointestinal tract
 - liver, pancreas
- tooth
- skin, hair
- central nervous system
- kidney
- muscle (satellite stem cells)

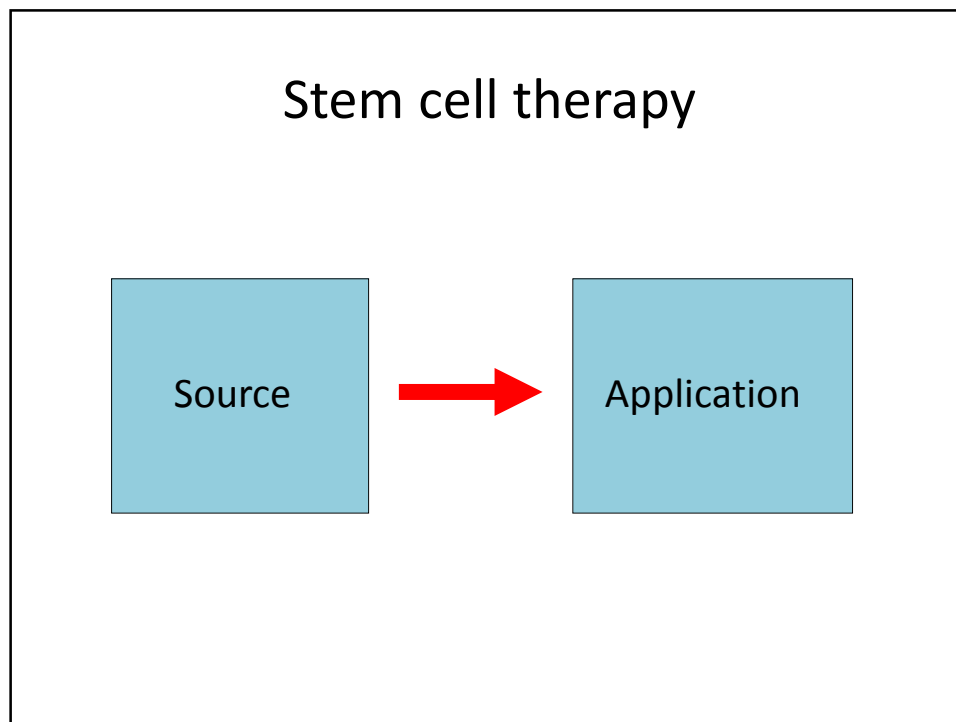


Embryonic stem cells

Method of obtaining ES cells:

- Blastocyst
 - 5 day old embryo
 - approx 100 cells
- Remove inner cell mass
- Cultured cells = ES cells
- Pluripotent





- ### Stem cell therapy
- #### Current reality vs. future promise
- Current reality
 - only a few well-established indications
 - Future promise
 - provides a source of hope for many patients
 - exploitation of emotionally-vulnerable patients
 - Ethical issues
 - embryonic stem cells

The Human Genome

- 3×10^9 base pairs from each parent
- 6×10^9 bp in the nucleus of every (nucleated) cell in the body
- If stored in book form:
 - 1 000 pages each with 1 000 letters
 - 6 000 books would be needed

When does a variant
become a disorder or a
disease?

Variants

- When does a variant become a disorder or a disease?
 - Subjective distress/suffering
 - Significant impairment of personal, social, occupational or other important areas of life
 - Death

Ethical, legal and social issues

- Informed consent
 - Emphasis on informed
 - Two tiered approach
 - Community
 - Individual
- Sample and data storage
- Privacy/confidentiality
- Access to data
 - Incidental findings

Ethical, legal and social issues

- Genetic discrimination
 - Discrimination against individuals
 - Discrimination against communities
 - Examples
 - susceptibility to disease
 - intelligence, personality
 - sexual orientation
 - ancestry/ethnicity

Who will benefit?

- **Everyone ! (should)**
- No single life is better than another
- The “haves” vs. the “have-nots”
- Need to embrace national, local, ethnic and gender-specific differences
- Prioritization of common vs. rare diseases

Responsible leadership

- Ethics and moral values
- Legislative framework
 - Ethics/moral values trump the law
- Translational research
 - Bioentrepreneurship
- Public dissemination