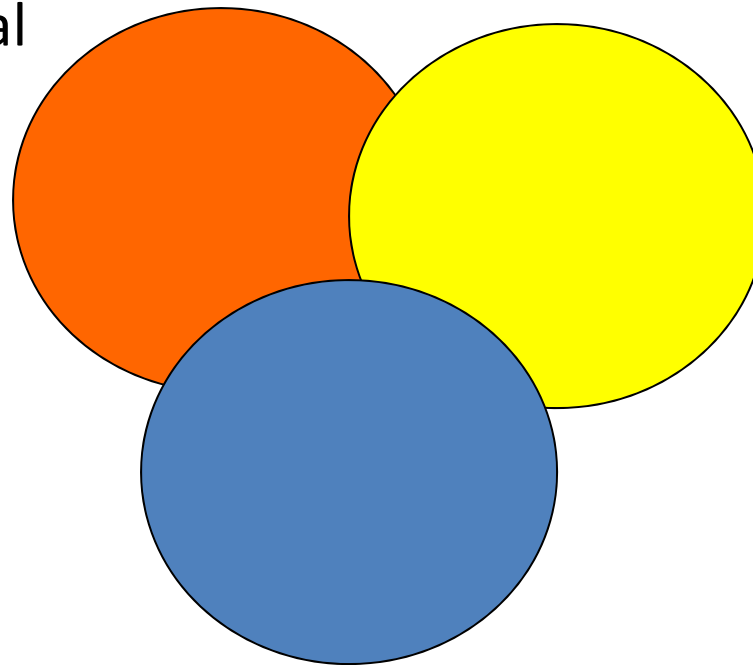


# Responsible and Irresponsible Medical Innovation with Stem Cells

Jeremy Sugarman, MD, MPH, MA  
Harvey M. Meyerhoff Professor of Bioethics & Medicine  
Berman Institute of Bioethics and Department of Medicine  
Johns Hopkins University  
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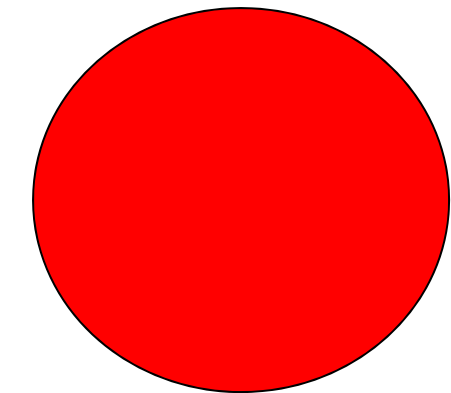
# Clinical Pathways

Translational  
Research



Innovation

Clinical Care



Inappropriate Use

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# Selected Conditions Treated

- Cancers
  - ALL, AML, CML
- Bone marrow failure syndromes
  - Aplastic anemia, Fanconi's
- Hemoglobinopathies
  - Sickle cell, thalassemia
- Inborn errors
  - SCID

# HDCT/ABMT or Breast Cancer

- Rapid dissemination of an innovative therapy offering hope
- >30,000 women received it before it was shown to be ineffective

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# THE LANCET

The Lancet, Early Online Publication, 23 October 2013  
doi:10.1016/S0140-6736(13)62033-4

## Groundbreaking Trachea Transplant Could Become Routine

Oct. 23, 2013

By KATIE MOISSE via [GOOD MORNING AMERICA](#)

*Science* 19 April 2013:  
Vol. 340 no. 6130 pp. 266-268  
DOI: 10.1126/science.340.6130.266

NEWS FOCUS

### Trachea Transplants Test the Limits



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IMAGE REMOVED FOR WEB POSTING

The NEW ENGLAND JOURNAL of MEDICINE

BRIEF REPORT

## Vision Loss after Intravitreal Injection of Autologous “Stem Cells” for AMD

The NEW ENGLAND JOURNAL of MEDICINE

BRIEF REPORT

## Autologous Induced Stem-Cell–Derived Retinal Cells for Macular Degeneration

NEJM 2017; 376 (11)

# Guidelines for the Clinical Translation of Stem Cells

- Multidisciplinary international task force convened by the ISSCR
- Released December 2008
- Updated May 2016
- Available at [www.isscr.org](http://www.isscr.org)

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# Intent

*...that basic stem cell research is responsibly translated into appropriate clinical applications for treating patients.*

# Overview of the Guidelines

1. Fundamental Ethical Principles
2. Laboratory-based Research
3. Clinical Translation of Stem Cells
4. Communications
5. Standards in Stem Cell Research

# Clinical Translation of Stem Cells

1. Cell Processing and Manufacture
2. Preclinical Studies
3. Clinical Research
4. *Stem Cell-based Medical Innovation*
5. Clinical Application

## *Provision of Innovative Care*

“Clinician-scientists may provide unproven stem cell-based interventions to at most a very small number of patients outside the context of a formal clinical trial and according to the highly restrictive provisions outlined in this section.”

(ISSCR, 2017, 3.4.1)



# Provisions for Innovative Care

- There is a written plan
- Plan is approved through peer review
- Patient not eligible for a trial
- Institution is accountable
- Personnel are qualified
- Voluntary informed consent
- Action plan for adverse events
- Resources for complications
- Commitment to contribute to generalizable knowledge

# Closing Comments

- Innovation has resulted in enormous benefits and enormous harms
- To minimize the likelihood of harm, innovation requires oversight