

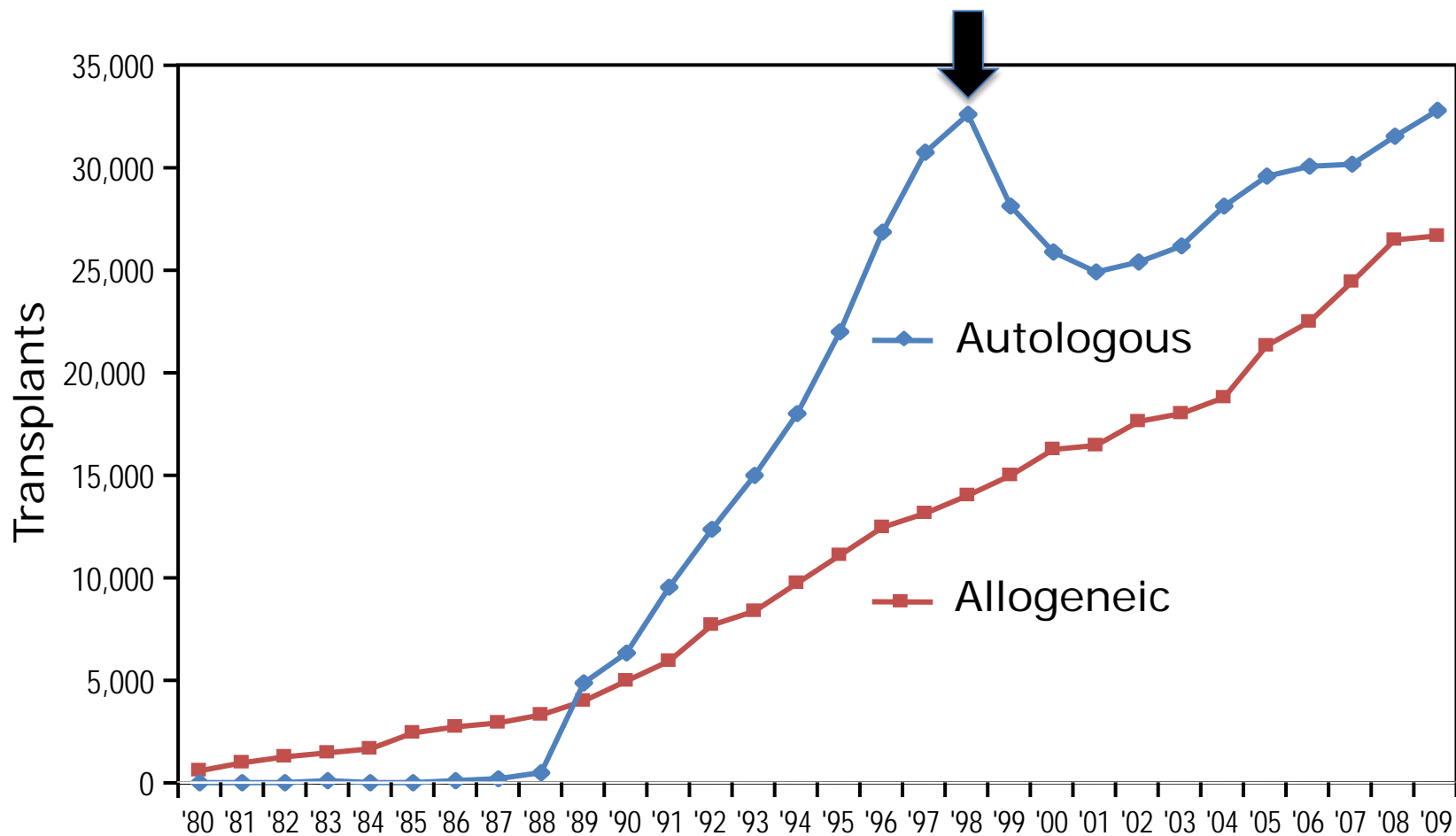
Professional Guidelines for Stem Cell Translation

ISSCR

George Q. Daley

Director, Stem Cell Transplantation Center
Children's Hospital/ Harvard Medical School
ISSCR President 2007-2008

HSC Transplant activity worldwide 1980-2009



International Society for Stem Cell Research

- Promote safe, effective, innovative clinical translation:
 - Educate scientists and medical practitioners world-wide
 - Establish professional standards
 - scientific, medical, ethical
 - Help inform, formulate regulatory pathway
 - Train translational physician scientists
 - Educate patients and their families
 - Spotlight clinics marketing unproven treatments

Why are stem cells different from drugs?

- Stem cell products are entirely novel
- Stem cells persist in the patient
- Animal models only partially predictive

2008

New ISSCR Guidelines Underscore Major Principles for Responsible Translational Stem Cell Research

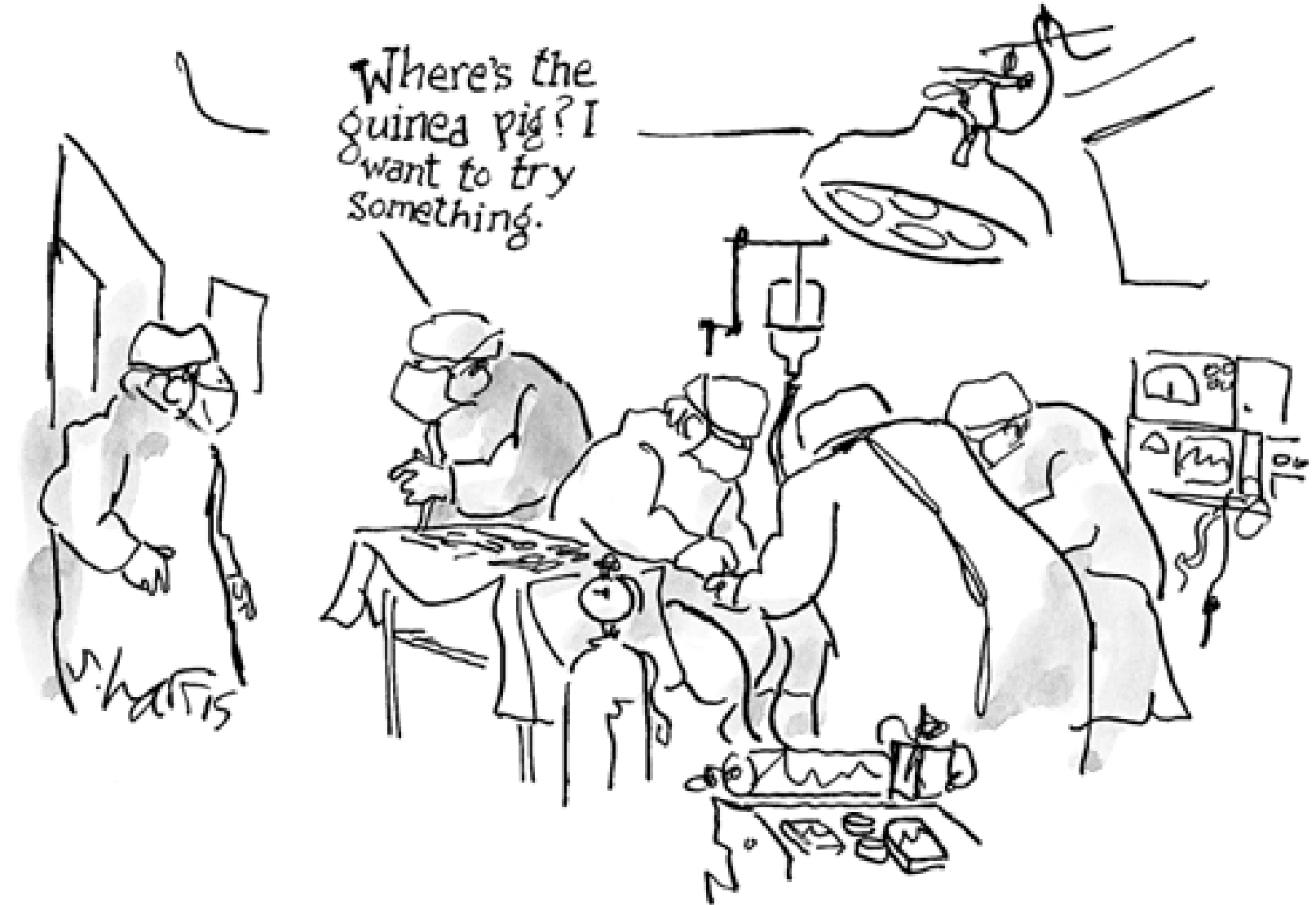
Insoo Hyun,^{1,*} Olle Lindvall,^{2,*} Lars Åhrlund-Richter,³ Elena Cattaneo,⁴ Marina Cavazzana-Calvo,⁵ Giulio Cossu,⁶ Michele De Luca,⁷ Ira J. Fox,⁸ Claude Gerstle,⁹ Robert A. Goldstein,¹⁰ Göran Hermerén,¹¹ Katherine A. High,¹² Hyun Ok Kim,¹³ Hin Peng Lee,¹⁴ Ephrat Levy-Lahad,¹⁵ Lingsong Li,¹⁶ Bernard Lo,¹⁷ Daniel R. Marshak,¹⁸ Angela McNab,¹⁹ Megan Munsie,²⁰ Hiromitsu Nakauchi,²¹ Mahendra Rao,²² Heather M. Rooke,²³ Carlos Simon Valles,²⁴ Alok Srivastava,²⁵ Jeremy Sugaman,²⁶ Patrick L. Taylor,²⁷ Anna Veiga,²⁸ Adrienne L. Wong,¹⁰ Laurie Zoloth,²⁹ and George Q. Daley³⁰

– Background: 30th anniversary, 10th book

- Scientists, physicians/surgeons, evidence, peer-reviewed
- Rigorous patient advocates around cell manufacture
- Scrupulous review of clinical protocol by IRB
 - Ensuring informed consent, patient autonomy
- Rigorous long-term follow-up, reporting of negative results



Medical innovation (outside formal clinical trial)



Declaration of Helsinki (10/19/13)

World Medical Association Declaration of Helsinki Ethical Principles for Medical Research Involving Human

Subjects **FREE** **ONLINE FIRST**

World Medical Association

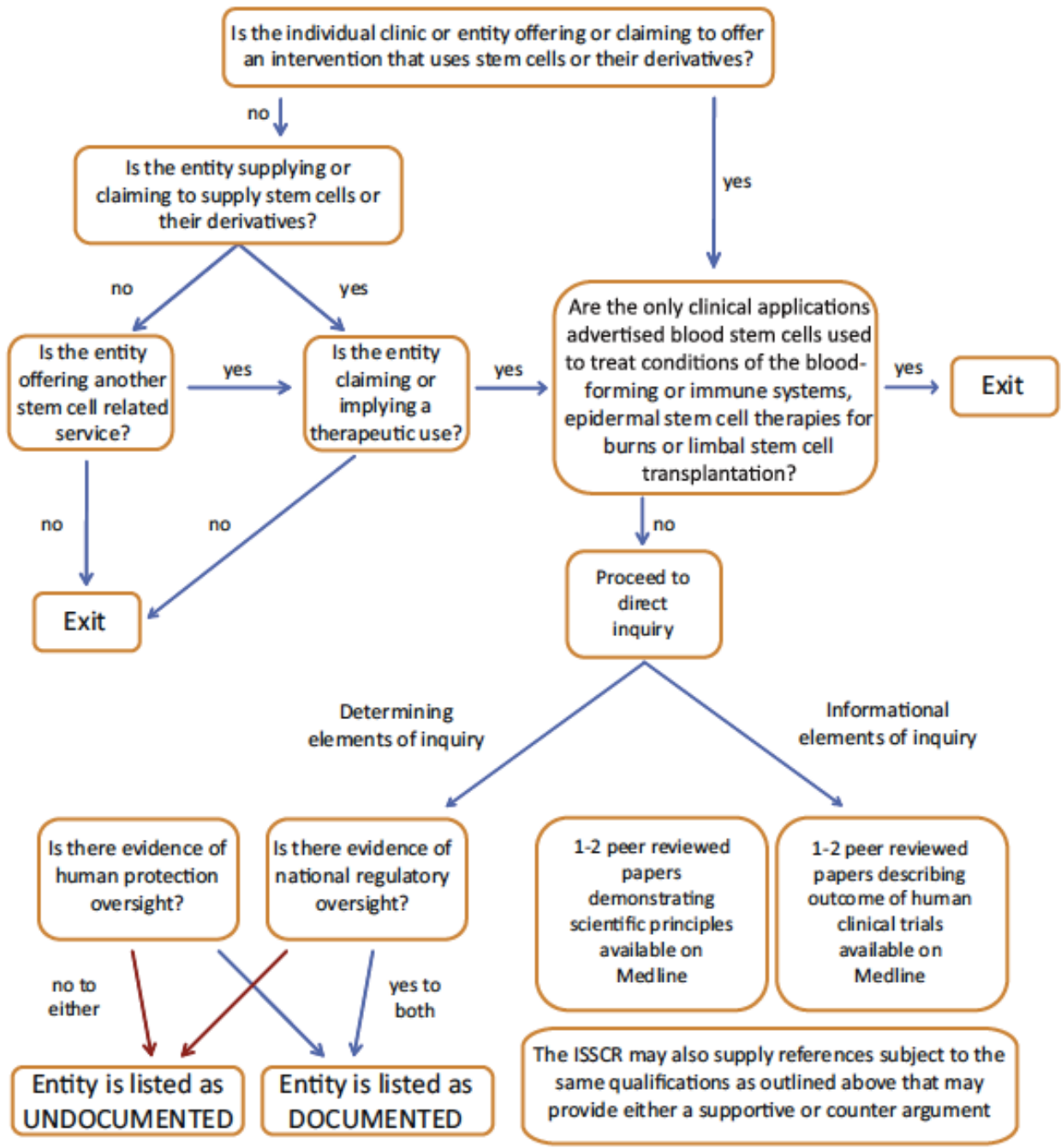
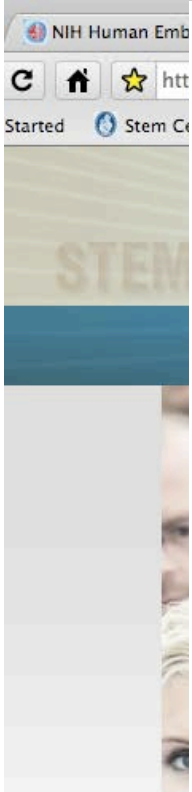
JAMA. Published online October 19, 2013. doi:10.1001/jama.2013.281053

Text Size: [A](#) [A](#) [A](#)

http://jama.jamanetwork.com/article.aspx?articleid=1760318&utm_source=silverchair+information+systems&utm_medium=email&utm_campaign=jama%3aonlinefirst10%2f19%2f2013

UNPROVEN INTERVENTIONS IN CLINICAL PRACTICE

37. In the treatment of an individual patient, where proven interventions do not exist or other known interventions have been ineffective, the physician, after seeking expert advice, with informed consent from the patient or a legally authorised representative, may use an unproven intervention if in the physician's judgement it offers hope of saving life, re-establishing health or alleviating suffering. This intervention should subsequently be made the object of research, designed to evaluate its safety and efficacy. In all cases, new information must be recorded and, where appropriate, made publicly available.



Cell Stem Cell
ISSCR: Rep
Patient
Stem C

Patrick L. Taylor,¹ Roger A. I
 Elena Cattaneo,⁴ Alan Colma
 Harold Edgar,⁷ Ira J. Fox,⁸ C
 Lawrence S.B. Goldstein,¹⁰ I
 Andrew Lyall,¹² Robertson F
 Fernando J. Pitossi,¹⁴ Emes
 Heather M. Rooke,^{16,*} Dougl
 Susan Stayn,¹⁹ Gary K. Steir
 Amy J. Wagers,²⁰ and Irving

GO
 search
 or
 reply
 to
 i.
 y
 ly."
 itute
 cal
 for
 ,
 n't
 stem
 and

Promoting ethical, effective care

- Community and Professional standards
 - Reinforced thru continuing education, practice guidelines, professional societies, peer consultation
 - Professional accreditation/ certifications
 - Practice standards and legal liabilities
 - Tort law and medical malpractice
- Regulatory oversight
 - Legal, governmental, institutional
- Market forces
- Need to address patient, family needs

