



AMERICAN INSTITUTE FOR MEDICAL
AND BIOLOGICAL ENGINEERING

AIMBE/NIH Summit on
**Validation and Qualification of
New In Vitro Tools and Models for the
Pre-Clinical Drug Discovery Process**

March 19, 2012

Lister Hill Auditorium, NIH Campus, Bethesda, MD



NATIONAL INSTITUTE OF BIOMEDICAL
IMAGING AND BIOENGINEERING

- Drug discovery costs too much and takes too long – especially pre-clinical
- How can AIMBE help with this problem?
- Advocate to get new technologies into the pre-clinical space to REPLACE existing methodologies – especially animals

- ...the next generation of systems biology tools will be in vitro systems that are in defined, serum free conditions and composed of human cells...

Lee Hood

Days of Molecular Medicine

Conference, May 7, 2009

- Geron Corporation and GE Healthcare establish agreement to create tools to test for toxic effects of drug treatments

June 30, 2009

- Establishment of Regulatory Science Initiative between NIH and FDA

February 24, 2010

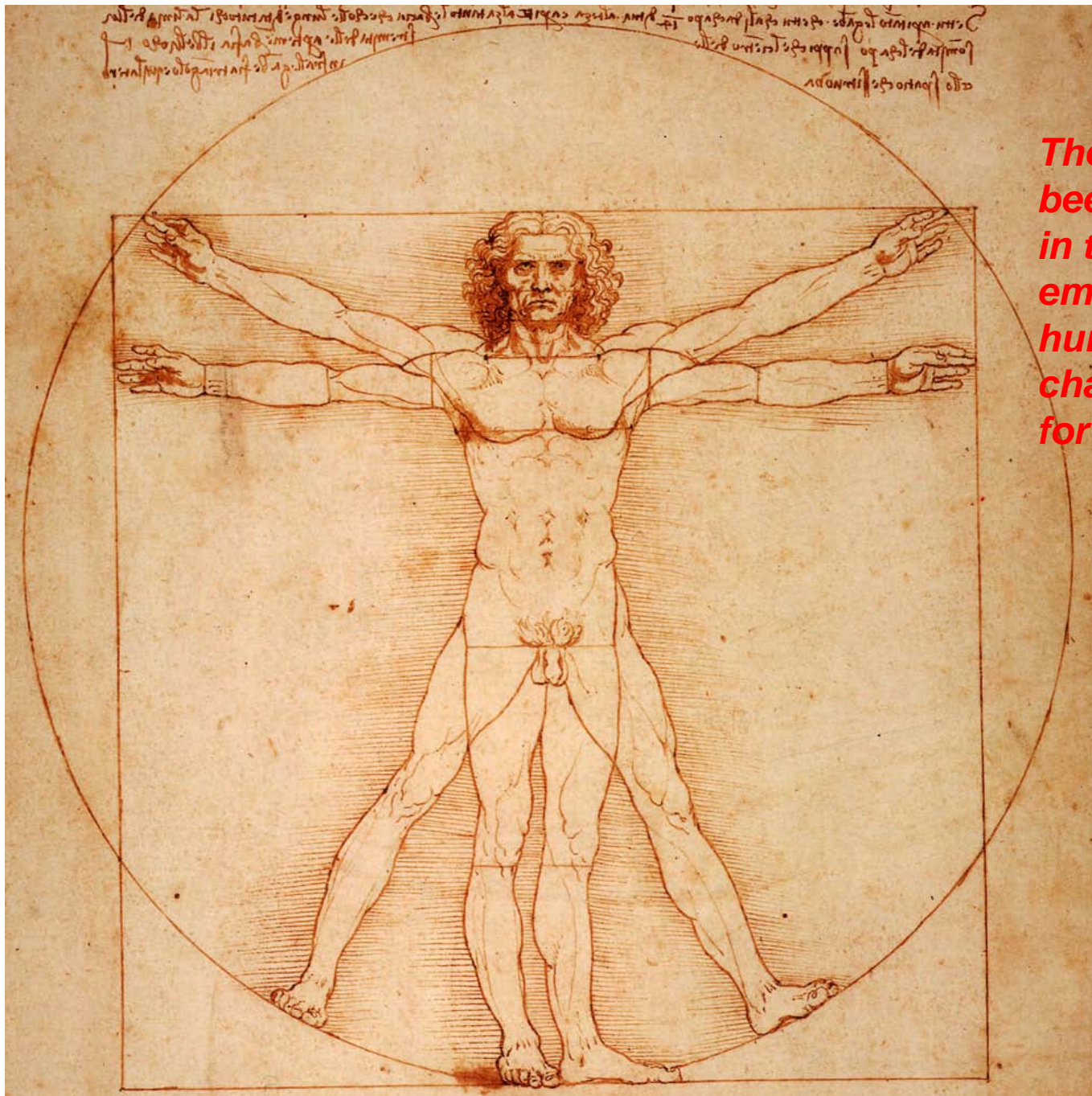
-with earlier and more rigorous target validation in human tissues, it may be justifiable to skip the animal model assessment of efficacy altogether

Francis Collins July 6, 2011

Science Translational Medicine

- Authorization of NCATS

February, 2012

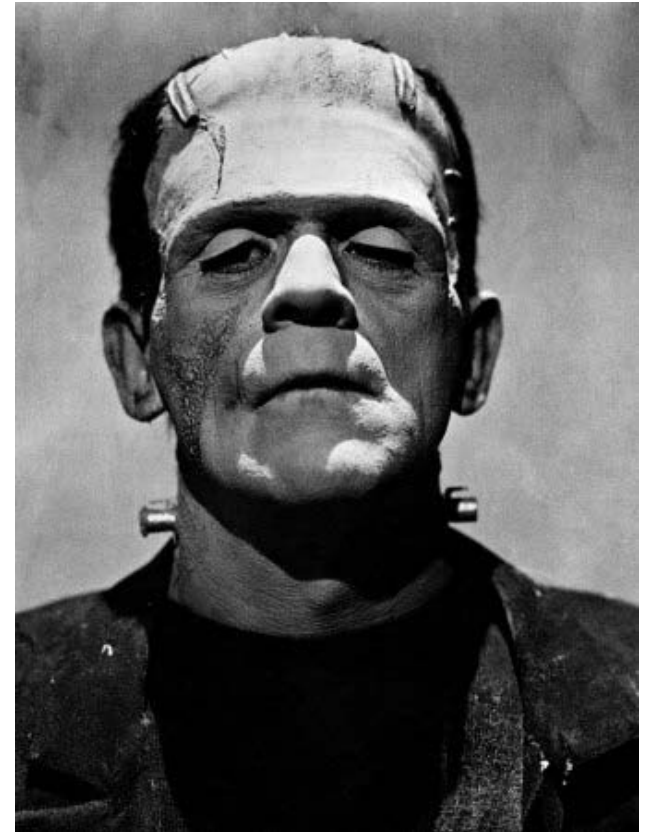


There has been interest in the emulation of human characteristics for centuries...

- However, although it seems simple after much discussion with the NIH, FDA and industry it's a far more complex issue
- Recurring topic was validation and qualification of new technologies – especially for replacement of existing technologies
- Needs community to come together to explore the technology and the definitions of *validation* and *qualification*

Current Situation

- Most systems in use today utilize single cell assays or animals →not much in between
- New systems being proposed to reproduce organs, hooking these together with vascular constructs but at the other extreme you get ...
- Need to explore combination of tissue engineering, cell biology, micro-systems technology, systems biology and predictive model systems



Goals of Summit

- Obtain definition of validation from across the spectrum of the drug discovery enterprise for preclinical platforms and systems.
- Define qualification from the perspective of the FDA for these systems.
- Gather as much data/input on this topic as possible.
- Use to define concrete goals for a workshop to be held in September, 2012.