## Translational Research

"Ask the Experts"
PACT Web Seminar
November 14, 2013

Catherine Matsumoto
City of Hope

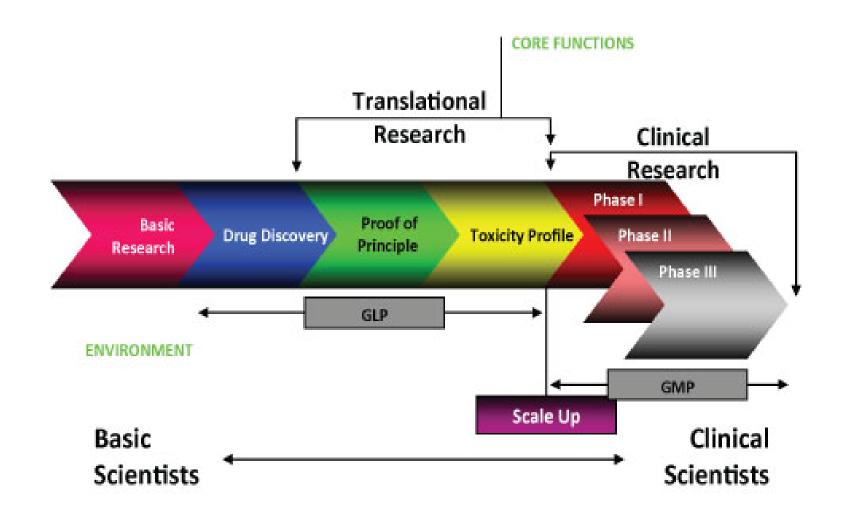




"It is the responsibility of those of us involved in today's biomedical research enterprise to <u>translate</u> the remarkable scientific innovations we are witnessing into health gains for the nation."

- Elias Zerhouni, 2003 as NIH Director

N Engl J Med 2005; 353:1621-1623. October 13, 2005



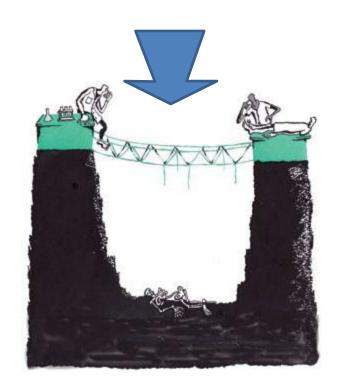
## **Discovery to Translational**

- Therapeutic candidate identified during the basic research and discovery phase
- Mechanism of action in disease model
- Early preclinical therapeutic proof of concept studies
- ➤ Identify unique/novel issues related to your product
- ➤ Identify assays needed to further characterize the therapeutic candidate
- ➤ Identify additional preclinical studies that should be performed to adequately assess safety

T1

- Clinical trials design
- Regulatory affairs
- Biostatistics
- Manufacturing
- Product characterization
- Pre-clinical studies

Bench



Bedside

The "Gap" (a.k.a. "Valley of Death")



"It's nothing a few stem cells and another 75 years of research can't fix"