



IOM Workshop

APPROACHES TO ACCELERATING TRANSLATIONAL SCIENCE



Changes to Biotech Startup Ecosystem

Venture Capital Funds Reduced Commitment to Early Stage/Translational Biotech

- Demand from “buyers” (IPO, M&A) increasingly requires more mature programs which put early stage Biotech projects beyond VCs’ investment horizon
- Perception of increased regulatory risk
- Everyone is chasing the next Facebook

Less Capital for Translational Programs

Aes-103: Multi-Stage, Multi-Institute, Public-Private Translational Program

- AesRx
- National Heart Lung and Blood Institute (NHLBI)
- Therapeutics for Rare and Neglected Diseases (TRND)
- NIH Clinical Center
- NIH Clinical Pharmacy

Major Milestones Achieved Rapidly

- IND filed in less than 1 year
- Completed healthy volunteer safety trial in less than 15 months
- First dose in Sickle Cell Patients in less than 18 months
- Successful Type C meeting with FDA to clarify clinical endpoints and regulatory pathway

Key Elements For Success

- Programmatic not episodic planning
- Quick, transparent decision making ability
- Genuine collaborative focus – good science is a necessary but not sufficient condition for success
- Flexible when necessary in response to both new programmatic insights and unfolding data

Key Elements For Success (cont.)

- Significant advantages of virtual model
 - Capital efficient, low overhead
provides more bang for the buck
- Industry partner brings Big Pharma-type clinical development expertise

Challenges to Success

- Government procurement process
- Diffuse responsibility makes tight budget control difficult

The Secret Sauce

- Focus
- Focus
- Focus

Important Results Can Be Achieved

“It only just now occurred to me the historic moment we witnessed yesterday:

The first dose ever administered to a sickle cell patient of a drug specifically designed to prevent sickling.

Welcome to a little piece of [medical] history in the making.”

Gregory J. Kato, M.D.
Intramural Researcher
Sickle Cell Vascular Disease Section
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