Obstacles to Translation Conference

IV. OBSTACLES TO RAISING NECESSARY MONEY

Bringing new drugs to patients is expensive and risky. Individuals and institutions will have different incentives for wanting to invest in discovery/development of treatments for rare monogenic skin disease. We all subscribe intellectually to the idea that development of molecularly targeted therapies not only will improve the lives of our patients but also will serve as models for the next generation of therapies for more common, genetically more complex disorders. Is this a truly compelling incentive?

a. What are the incentives that drive individuals and institutions to donate/invest? Given what we are trying to accomplish, are certain individuals or institutions more likely than others to come up with the cash? i.e. are the specific places where we should focus efforts to raise money?

b. What incentives will be needed to encourage individuals or institutions to accept the cost and risk of drug development for rare monogenic skin disease? What can we do to identify incentives that will drive acceptance of risk and expense?

c. The “someone else should do it” problem: Can/should donors be encouraged to collaborate on goals? If the research community agrees that delivery problems cut across all diseases under consideration, who should fund research in delivery?

d. Has Balkanization (focus on specific diseases) helped or hampered efforts to identify major donors to this effort?

I. Problem: Describe the Current Condition

- We need more money.
  - To bring basic science into research on humans.
  - For compound development.

I. Problem: Describe the Current Condition

- “You need money to get money”
  - Amount of data required to raise funding is growing, human data needed as well.
  - $5-10 million to develop the necessary data to attract investors.
  - Investors not willing to assume the risk at the early stage.
  - ‘Angel investors’ may contribute a smaller amount. Difficult to build on donations alone.

I. Problem: Describe the Current Condition

- Investors are looking for good management- academic investigators not perceived as good managers.
- Venture capital firms will start a company based on management and then look for scientific ideas to fill a void or concept.
- Orphan designation is not perceived as profitable, but can be used as a ‘stepping stone’ to more profitable ventures. This abuse of the system may cause mistrust.
- Risk of failure and production costs are high.
- There is fragmentation of intellectual property.

II. Analysis: Identify Possible Causes

- No grant money to bridge the gap between molecular and human work (ex. animal studies).
  - Reluctance from investors (risk-averse)
  - Market for orphan diseases not large enough, although orphan designation may be attractive to investors
  - Government not investing in translational research, as they do not recoup that investment
  - SBIR grants are one possibility

II. Analysis: Identify Possible Causes (cont.)

- Production costs are high- wages, salaries, regulations.
- Risk of failure is too high.
- Fragmentation of intellectual property.

III. Approaches: List Possible Strategies

- Problem: Not enough money
  - Seek investors, donors, or foundations with personal interest or who simply care about the disease. AAD as one possible source.
  - SBIR program available only to companies. Need to develop similar program for academic investigators or institutions.
  - Can there be creative models outside capitalism?
  - Cluster the separate competing efforts of different academic centers
  - More advertising and public awareness
  - Outsourcing to other countries

IV. Action: Determine Specific Next Steps

- Consider forming a for-profit dermatology company focused on rare diseases
  - Define mission statement
  - Create business model, name leaders in science and in management (well-known names to attract interest)
  - Pool smaller disparate efforts in rare genodermatoses
  - File for SBIR grants with independent investigators.
  - Designate registries and nationwide treatment centers for trials

IV. Action: Determine Specific Next Steps (cont.)

- National Endowment for the Treatment of Rare Disorders- to fund development of products for rare skin disorders.

- NIH funded center to develop infrastructure for therapy of orphan genodermatoses.
  - Lower production costs by creating economy of scale.

- Rare Disease Investment Fund