Obstacles to Translation Conference

III. OBSTACLES IN OUR VARIOUS INSTITUTIONAL CULTURES

Academic, corporate, health delivery, private foundation and governmental institutions have distinct missions and different systems of rewards. While each institution places its own relative value on respect, influence, power, position and money, individuals are rewarded most often because they command large constituencies, large budgets or because their work has great "impact" or novelty. Views of collaboration, openness, acceptance of risk, and acceptable timetables for evaluation of success can be quite different in the various institutional settings. Within these various institutional cultures lurk few overt but many covert obstacles. The Orphan Drug act and the FDA are notable in that they recognized obstacles which their actions presented to the development community and then made adjustments to reduce those obstacles.

a. Which institutional obstacles are significant impediments to developing drugs for monogenic diseases?
b. How might these obstacles be removed, reduced, or avoided?
c. Will development of drugs for monogenic skin diseases be more likely to succeed in one institutional setting rather than another?
d. Is an altogether different institution needed?

Or more specifically:

a. What are the obstacles at the FDA?
b. In academia?
c. In the corporate world?
d. At private foundations?
e. Is one institution better suited for this kind of work than another?
f. Individual/professional obstacles: Is working on a rare disease an obstacle to personal advancement, and therefore an impediment to recruiting and retaining the talent needed to develop these treatments? Is collaborative work an impediment to personal advancement? Are the timetables for competitive, peer-reviewed funding (and professional advancement in academia) realistic for drug development?
g. Intellectual property issues: Do IP issues prevent from getting needed collaboration or reagents?
h. Setting priorities: Is the institutional culture nimble enough to promote timely and effective evaluation of results and resetting of priorities?
i. Health delivery: Is our current US health delivery system an impediment relative to a national health system?

I. Institutional obstacles

- Which institution is the right place for translational research?
  - Assume academia is a valid place (at least initial stages)
- Is translational research done by individuals/experts in a monogenetic disease? Or to break down the “translational research roadmap” to teams
  - Assume that one expert can play a large part (example BioMarin)

I. Institutional obstacles in Academia

- Lack of training across spectrum of basic steps for students/post-docs/PI's
  - Lack of rewards/culture
  - How to file patent, protect IP, get GMP, how to file a physician IND, find resources, assemble team
- Protect IP
  - Recoup patent costs: Existing product (IND)
  - New product patent filing
- Need: to get clinical data and before VC's will invest
  - No funding and no infrastructure for pre-clinical studies (animal to phase
- Regulatory problems in academia
  - Out-licensing problems
  - Conservative regulatory agency and university office
  - Pain of IRB

II. Analysis: Identify Possible Causes

- Training:
  - Under-recognition of complexity of translational research
  - Lack or recognition of importance of application rather than just discovery
- Career path for translational researchers is not clear and complete
  - Tenure based on publications not translational attempts
  - Industry suspicion by academia
  - Inadequate resources and access to mentors/advice at critical junctures to help bridge interactions between academia and industry
  - Need help with GMP and clinical trials

III. Approaches: List Possible Strategies

- Institutional change
  - Value team approach and collaboration, multiple labs working on one disease
  - For the PI and lab, define a percentage of time for translational research (French model)
- Reward change
  - Patents equal publications in terms of reward system (promotion and study section)
  - Recognition for Co-PI's on grants

III. Approaches: List Possible Strategies

- Beyond academics:
  - More funding for Orphan diseases, ie, help to pay for patent filings
  - Easier access to library of resources or facilitator/mentor who has been through the translational process (through the patient advocacy groups)
  - Identify the “gurus” and teach the rest of us how

IV. Action: Determine Specific Next Steps

- Training changes
  - Formal training:
    - Masters in medical science to increase access to clinical exposure for PhD students
    - Coursework in biostats, clinical trials for MD students and young investigators
  - Mentoring during key stages in translational research, co-mentors in academics and industry
  - Funded step wise training across spectrum of clinical/translational research for investigators:
    - GMP training: David Woodley (for example)
    - Regulatory training: Alexa Kimball (for example)
- Institution changes
  - Change the isolation of individual PI's or single institutions (French and European models)
  - Core facilities in academia to share resources
  - Or Core facilities shared among multiple universities (facilitated by Foundations?)
  - Reduce regulatory redundancy (local, IRB, GCRC, FDA review)

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

- Reward change
  - Patents equal publications in terms of reward system
  - Co-PI's on grants
- Funding
  - Private philanthropy to fill in the gap between animal studies to POC in humans
  - Expand scope of funding/advocacy of SID/AAD to include Office of Orphan Products and Office of Rare Diseases
  - Recoup patent costs: better OTL, SID and AAD to help finance, patient advocacy groups (NORD, Genetic Alliance, Coalition of Skin Diseases)