



Obstacles to Translation Conference

I. OBSTACLES TO DRUG DISCOVERY

Unlike many of the drug discovery programs undertaken by big pharma, we are fortunate in that we start with clinically well-defined diseases and we already know the specific molecular targets. Why are we not testing these target molecules in high-throughput screens?

- a. Have we failed to develop valid screening assays? If so, is it because of lack of motivation/incentive? Lack of expertise? Lack of hardware? Lack of manpower?
- b. Is our access to chemical libraries limited? If so, why? Do we know where they are? Are there institutional obstacles to their use?
- c. Do intellectual property issues prevent queries of existing (private) databases that might contain clues to interesting lead compounds?
- d. Do investigators and institutions view this as unrewarding work?
- e. Do interested parties (investigators, investors, proprietors of physical and intellectual resources) know how to find each other?

I. Problem: Describe the Current Condition

- No interest from big Pharma for rare diseases
- Challenges for topical delivery of drugs (especially non small molecules)
- Academia requires education about the drug discovery process
- High Thruput Screens (HTS)
 - Initial hits are far from therapies
 - Extensive chemistry & \$ required for next steps

II. Analysis: Identify Possible Causes

- Rare genetic skin diseases may not be treatable by traditional approaches
- Lack of mature platforms for non-traditional approaches (RNAi, gene therapy)
- Lack of interest and funding for large efforts
- Lack of collaborative infrastructure between academia and industry

III. Approaches: List Possible Strategies

- Small Molecules, biologics, siRNA/antisense, gene therapy
- Expand utility of a target to additional indications
- Better understanding of the biology of the diseases allowing alternative therapy development
- Increase communications and networking – more meetings between small biotech (novel technologies) & academics
- More academic rewards for later translation (not just initial ‘splash’)

IV. Action: Determine Specific Next Steps

- SID/ESDR establishes an award for ongoing translational research accomplishment
- SID/ESDR host scientific session/retreat and or reception to increase academic and industry interactions
- Engage patient groups for funds & political influence
- Rare diseases as a paradigm for tx in more prevalent diseases
- (other groups suggestions combined:)
 - Focus resources via a biotech startup on a single selected genetic skin disease and ultimately a single technology.