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HST.939 Designing and Sustaining Technology Innovation for Global Health Practice
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Lecture 10: 4/8/08 Biopharmaceutical Innovation

I. Intro

Going into the discussion, think about:

- ROI for industry (figures change based on what industry you're looking at) (e.g. \$800 million, 8 years)
- Neglected or neglected (e.g. existing conditions such as CVD, diabetes, cancer)
- Infrastructure (testing and clinical trials)
- Innovation incentives (e.g. advanced market commitments)

Home stretch

- today is the first of the deliverables and mentorship sessions
- last few weeks of class

II. Lecture by Joanna Lowell

Overcoming the Global Health Innovation Gap: A role for the biotechnology industry in drug discovery for neglected diseases

1. Overview of BVGH
2. The problem: how to go about creating new medicines for neglected diseases? (e.g. malaria, TB)
3. Innovation map project
4. Effort to create incentives for industry's involvement in global health

1. Overview of BVGH

- small company funded by Rockefeller Institute
- mission is to figure out how to get biotech industry engaged in making vaccines, diagnostics, treatments for global health, neglected diseases
- work through advocacy and education
- approach is threefold: drive funding, build strategy, and create new information and opportunities

Creating a product pipeline for neglected diseases: wish it were as simple as having a good idea and piping it into the market. Reality is, there are many barriers to getting that idea implemented in the marketplace/real world. BVGH tries to remove those barriers.

One billion out of six billion people have access to quality health care, globally. New products could make a difference.

- There are practically no vaccines for parasitic diseases.
- We need diagnostics, vaccines, and drugs to combat bacteremia, malaria, sleeping sickness, etc.

- 90% of biopharm industry's R&D is spent on problems that affect 10% of the population.

PDPs (product development partnerships): basically they're nonprofit, virtual pharm companies. Most focus on one or few diseases in particular.

- goal: 1) create a situation with robust, sustainable pipeline resulting in new drug every 3-5 years; 2) create new drugs that meet idealized target product profiles (TTPs). Examples of desired "breakthrough" therapies include:
 - o TB: cure active disease <1 month
 - o Malaria: single dose cure
 - o Etc
- there are some success stories already, so this model can work

2. The problem: how to go about creating new medicines for neglected diseases?

Process of drug development: essentially, there's a discovery phase, and a development phase. Typically requires 10-15 years, and is very expensive (partly because of how long it takes). Almost everything that enters the pipeline fails (e.g. you may start off with 5,000 compounds, and end up with 1 that actually works).

2/3 of small-molecule compounds in clinical trials in 2007 originated with biotech companies. Yet the biotech companies are largely absent from neglected disease drug discovery and development; large innovation gap. Academics and PDPs are moving into the gap but this hasn't made a large enough difference.

3. Innovation Map Project

- Map the capabilities of the biotech industry against unmet therapeutic needs for neglected diseases.
- Essential discovery tools have been created for mycobacterium, plasmodium, and trypanosoma
 - o Genomes sequenced for multiple species
 - o Detailed understanding of metabolic pathways
 - o Gene knockout tech
 - o Whole-cell assay and biochem screens
 - o Adequate animal models

Companies are organized around targets

Key Findings:

- innovation gap in drug discovery for malaria, TB, and HAT
- science sufficient to support discovery
- biotech well positioned to lead discovery
- technologies and drug targets are transferable
- substantial new investment required
- value justifies investment and required risks

Barriers to biotech participation:

- information:
 - o unfamiliar with neglected diseases
- funding:
 - o skeptical of investor support for global health;
 - o unable to access sufficient R&D funding;
 - o concern about opportunity costs and focus
- markets:
 - o unsure of developing work markets;
 - o disbelief in profit to be made

4. Market motivates industry; drives R&D priorities

Products for neglected diseases have comparable (equal or even greater) costs but lesser returns than for diseases found in the developed world

Need for market-based approaches

Push mechanisms:

- reduce risk and cost for R&D
 - o PDPs
 - o Grants
 - o Product development fund

Pull mechanisms:

- enhance market opportunity

Best incentive, though, is a real market.

- purchasing power based on market need
- sustainable
- independent of external intervention
- less to no need for subsidy
- less likely to depend on single customer
- *example: Rotavirus vaccine*

Even if a market is there, lack of awareness is the key barrier to entry.

The next best thing to having a market is creating one.

- Public sector purchases products.
 - o Builds markets for existing products
 - o Strengthens delivery systems
 - o Stable, growing funding attracts innovators

Advance Market Commitments (AMCs)

- Features:
 - o Binding, forward contract ensures market for new products
 - o Guaranteed price

- Funding by gov'ts of 1st world countries
- Benefits
 - Only pays for success
 - Rewards R&D investment
- Issues:
 - Hard to predict investment costs
 - Success judged by IAC

Priority review voucher (PRV)

- - - - - end

III. Lecture by Gail Cassell, VP of international affairs at Ely Lilly

Tuberculosis still infects 1/3 of the world population

CDC and WHO formerly advised not to treat MDR (multiple drug-resistant) TB, until a small group of doctors showed that a cocktail of drugs (including a couple produced only by Lilly) could cure MDR TB in 86% of patients. CDC and WHO changed their guidelines. This prompted Lilly to provide funding to CDC and the doctors to allow continued treatment and monitoring of the situation. They also wanted to transfer knowledge of how to manufacture these two old drugs to 4 affected countries and a university.

This is the largest philanthropic effort in Lilly's history. They have no other infectious disease products or initiatives.