

The Role of Science and Business in Maturing Concepts into Medical Products

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International BioScience

Overview

- Pharmaceutical Industry
- Biotechnology
- Startups
- Investment Community
- Medical Products
- Validation of Medical Products

Pharmaceutical Industry: Selling products

The Pharmaceutical Industry

- *Pharmaceutical Industry*
- International
- National
- Biotech companies
- Start up companies
- Entrepreneurs
- Concepts/Visions
- Patients/Physicians
- Insurers/Uninsured
- Government:State/Federal
- **Pharmaceutical Industry**
- Health Care Institutions
 - Managed/Non-Profit
 - National/ International
 - \$\$\$ drug pricing*

Origins of the Pharmaceutical Industry

1880's Germany

Government, Business and Academics

Education; Technology Colleges

1930's England

Emigration of German Scientists

Penicillin,* Government and Academics only

* (The Mold in Dr. Florey's coat by E. Lax)

World War II

US Manufacturing in WW2

- US Government & the Pharmaceutical Industry
- Manufacturing of Penicillin
- Fermentation plants in the Pharmaceutical Industry produced kilogram quantities of Penicillin for the war effort.
- Penicillin became a wonder drug made on an industrial scale.

Why Drug Development in the US?

1945 to ~1980

- The College GI Bill sent veterans to college.
- US Government development of the Medical Centers and Universities by the NIH (\$\$\$)
- The war on Cancer (1970's) \$\$\$
- European Scientists attracted to the US Medical Centers by the scientific resources and funding available.

The Pharmaceutical Industry Today

Consolidation?

~ 3-5 big Pharmaceutical Companies

or

Niche Markets ?

Genetic diagnostics markers and
therapeutics for a specific class of
patients (i.e. Herceptin, Genentech;
Breast Cancer)

Biotechnology Industry: Making products

Why did Biotechnology start in California?

- 1970's Biotechnology
- Money (Investment Banking) meets Science (Molecular Biology).
- Why San Francisco? Germany 1880?
- Why not New York? US? Europe? Asia?
- Academics, Business and Government

Why Dr. Boyer in ~1975?

- Was Dr. Boyer the first to be approached?
- Why an academic at UCSF?
- Why was Insulin was the first product?
- What is the attitude in Academics today?
- Ask the Technology Transfer Office.

Insulin

- What was the source of insulin prior to genetically engineered insulin?
- Why was it not synthesized at Genentech?
- What was the impact of this product on the government, business and academics?
- “The Eight Day of Creation” by H. Judson (Molecular Biology from 1940-1980)

2010?

- **Advances in Biotechnology tomorrow (2010)?**
- Will it be in Europe?
- Will it be in Asia?
- Will the US continue to dominate the field?
- **Government, Business and Academics?**
- Stem Cell Research?
- Education (Technology Colleges of the 1880)?
- Digital information, live internet conferences!

Startups: Validating Concepts

Start-up Essentials

- Create & Sustain breakthrough technology
- Value Proposition
- High growth market
- Secure “marquee” customer early
- Build alliances with major companies
- Fast Revenue growth & high ROI
- Management team & Board

Successful Early Stage Companies

- Innovation
- Commercialization
- Business model:
 - science, IP, competition, manufacturing
 - customer, market size, financials,
 - management, board, advisors
 - deal structure, exit strategy, risks, ROI

U.S. Entrepreneurs

- **Deal Flow:**
- Concept/vision
- Funding: Friends, Family and Fools (\$250,000b)
- Angels: early stage validation (\$.25-2 million)
- Funding Gap: \$2-5 million
- Venture Capital: \$5million and above
- IPO or Buy out

Investment Community

Angel Investors

- 2000-2005

Stronger: Mentoring, Due Diligence, ROI

- ~200 Angel Groups in the US; ~20,000 Angels
- Largest “Angel Group” is in Southern California
- Tech Coast Angels: SB-San Diego (~270);
- Pasadena Angels (~100)
- Angels work closely with Academic Institutions

Angel Capital Overview

- Invested ~\$15.7 Billion in 2002 in ~36,000 ventures
- Historically: ~\$30 Billion in 500,000 ventures with about ~400,000 Angels
- Invest annually: ~\$300 million/year

Why Angels exist?

- First round capital is hard to find
- Management talent is a scarce resource
- Venture capital funds have grown dramatically
- VC investments are a minimum of ~\$5 m
- VC startup deals are only ~2-5% of their total deals.

Angel Investor Characteristics

- Invest their own capital
- Limited financial resources
- Variety of Professional Backgrounds (i.e. CEO, COO, CMO, CSO)
- Well connected with networks & resources
- Invests close to home
- Patient capital

Angel Value Add

- Funding –early and later VC rounds
- Great networking source
- Mentoring
- Board of Directors & Advisory Board
- Experience founding and building a company
- Active investor

Funding Process

- Initial contact through the website
- Pre-screening
- Screening
- Mentor and/or Champion
- Presentation to the full membership
- Due Diligence
- Funding; individual angels invest

Investment Criteria

- Does the company address an important problem?
- Does the company offer a good solution?
- Do many people have this problem?
- What is the competition?
- Has the company made good progress?
- Is there are sound financial plan for the future?
- Do they have the management team to execute the plan?
- Will the company produce reason able returns for the investor?

Spreading the Risk

- Entrepreneurs
- Angels and Venture Capital Groups
- Biotechnology Sector
- Pharmaceutical Industry:
 - Is this model unique to California? US?
 - Europe? Asia?
- Government-Business-Academic Institutions

Medical Products

Therapeutics: (selective and non-toxic)

New Chemical Entities (NCE): small molecules

Biologicals: MCA, Vaccines, Proteins, Gene Therapy, ...

Diagnostics (In Vitro/ In Vivo)

Nutritional Supplements

Medical Devices

How do you make a Medical Product?

The Pharmaceutical Development Pipeline:

- Pre-Clinical Research, validation of the science
- Clinical Development, Regulatory/CMC
- Project Management, Product Launch
- Management, the senior team and the board
- *Pharmaceutical Development-*
1st year course (30 lectures/90 hours).
Biotechnology Center, Dublin, Ireland (20 hours)

Why do Medical Products succeed?

- It meets an unmet medical need.
- It is pure, safe and efficacious.
- The product adds value to the company.
- It can be a disruptive technology.

Biotechnology

- 1985-2000;
- 137 medical products in the market
- ~1900 failures
- 2005
- 1400 Biotech Firms;
- 350 drugs in development

Why do drugs fail?

Selective and Non-Toxic (ADMET)

A: administration of the drug to the patient

D: delivery of the drug to the target tissue
(PK and PD)

M: metabolism of the drug in the cell

E: efflux of the drug from the cell/tissue

T: toxicology

Case Studies

1. Science/Technology
2. Clinical Development/FDA
3. Management
4. Manager from the Company

How to Analyze a Medical Product

The Pre-Clinical Science:

Does the science/technology fill an unmet medical need?

Define the science/technology of the product and its utility.

Is the science/technology novel and validated?

What is the non-human pharmacology/toxicology?

Has the medical product been manufactured (pilot batch)?

Intellectual Property: Is it filed/issued/challenged?

What other products are in competition with this concept?

How to Analyze a Medical Product

The Clinical Development

Does the clinical data meet an unmet medical need?

Are there manufacturing issues for scale up of the product?

What is the human pharmacology/toxicology (Phase 1)?

What is the indication and is it efficacious (Phase 2)?

What is the optimal dose for the specific patient (Phase 3)?

FDA: Is the medical product pure, safe and efficacious?

FDA: Fast Track Approval for an unmet medical need?

What are the competing clinical products in this field?

How to analyze a Company

The Business

What is the track record for the management team?

What is the financial strategy for the company/product?

What is the sales & marketing strategy for the product?

What are the other competitive products in the field?

What is the market size and expected penetration?

(locally, regionally, nationally and internationally?)

What are the expectations of this product in the market?

(6, 12, 24 and 48 months?)

Summary

- The process from “concept to market”
- Medical product: Validation, Scale up, Clinical, Sales & Marketing
- What is the role of an early investor?
- Biotech: Validation of the Market
- Pharmaceutical Industry: Distribution
- Role of Business, Academics and Government

Ireland: Yesterday and Today

Ireland did not experience:

- Roman Rule
- The Reformation
- The Enlightenment
- Industrial Revolution

Ireland's advantage:

- Member of the EU
- An Adroit Economy
- College Educated
- English speaking
- Opportunist Culture
- Digital Information Age
- Globalized Economy

My Career

- **Academic Medicine**

- Molecular Biology (Univ. of London; Ph.D.)
- Cancer Biochemical Pharmacology (Yale Med. School)
- Clinical Cancer Pharmacology (Mt. Sinai Med. School)
- Research: Anti-Oncogenes Ribozymes & Gene Therapy
- Publications, Awards, Editor, President (ISCGT)

My Career

- **Pharmaceutical Industry**
Berlex; Schering AG; Berlin, Germany
- **Graduate School**
Science/Business course on the
Pharmaceutical Industry
- **Venture Capital Community**
Mentoring and Funding Entrepreneurs