Biotech Pharma Company Analysis

Drug pipeline

A **drug pipeline** is the set of drug candidates that a pharmaceutical company has under discovery or development at any given point in time.

This involves various phases that can broadly be grouped in 4 stages: discovery, pre-clinical, clinical trials and marketing (or post-approval). Pharmaceutical companies usually have a number of compounds in their pipelines.

The drug pipeline is an important indicator of the value and future prospects of a company. Usually the more compounds in the pipeline, and the more advanced stage that these are in the better.

Other factors that are taken into account when assessing the value of a pipeline include the size of the target market of each drug, the market share that the drug is expected to capture and the risk that it will not be approved.

The cost of developing a new drug is astronomical – typically a drug costs many hundreds of millions and can reach 1 billion dollars over 15–17 years. Assessing this risk and filtering out as early as possible compounds that may not eventually get approved is essential to the pharmaceutical industry and involves checking the effectiveness of drugs as well as the likelihood of toxic events (Adverse event prediction).

R & D pipeline Tool:

Spotfire Tibco Pharma Pipeline Analysis Software looks at the R&D pipelines of the top 20 global pharmaceutical companies

Start your **due diligence** on any biotech stock by asking their IR department the same five questions:

- When was the last time the company raised money (diluted) through a stock offering?
- How much cash (not cash equivalents) does the company have?
- What and approximately when is the company's next known catalyst?
- What is the company's quarterly burn rate?
- Does the company have an existing line of credit and if so how much can they draw on it ?

For upcoming PDUFA dates, Phase 2 and 3 trial data releases and links to PRs all sorted monthly and quarterly.

For FDA Calendar: www.biopharmcatalyst.com/fda-calendar

Therapeutic Areas:
STEPh Inc. develops therapeutic area reports (termed STEPh REPORTS) on drugs in research across multiple disease states. Reports can be tailored to individual client needs and are always kept current as new information becomes available. STEPh REPORTS are available for purchase on a 12-month subscription basis.
For Pipeline Tables: www.stephreports.com/news/category/company-news/

General Health News:
www.reuters.com/news/health

Learn more about Pharma-Biotech Product & Company Valuation:
www.celforpharma.com/pharma-management/training/pharma-product-company-valuation-course.com

FDA Approval Process

The FDA Drug and Biologic Approval Process: A hot topic

The high cost of prescription drugs is a hotly debated issue and poses a significant burden to individuals, families, and the federal government. Drug costs have led to **Medicare Part D**, a federally subsidized program meant to defray the costs of prescription drugs, particularly to the elderly. Though there are many contributing factors drug prices, their expense is due in part to the cost involved in completing the FDA approval process.

In order for pharmaceutical and biotech companies to market their drugs and biologics, companies must receive **FDA approval**, a rigorous, expensive, and time consuming process that can take over a decade to complete. **Of 5000 compounds discovered in the pre-clinical stage, only about 5 will make it through the entire FDA approval process. Therefore,** companies have to cover not only the cost of successful development of a single drug, but of many drugs that never make it to market.

The Phases in the FDA Approval Process

Pre-Clinical Phase

In the pre-clinical or *drug discovery phase* of the approval process, researchers look for potential new compounds to treat targeted diseases. Once a compound has been identified and refined to a formula that can be tolerated by humans, its toxicology is tested in animals and living tissue. The process takes roughly three and a half years. During this phase researchers look for:

- correct dosage level
- how frequently it should be administered
- the best delivery system (oral, topical, intravenous, etc.)

- short- and long-term survival of the animals

After pre-clinical testing is completed, the company then files an Investigational New Drug Application (IND) with the FDA. **Fast Track Designation** is an expedited review of a drug that is given to a company whose drug or biologic makes both a product and a marketing claim that addresses an unmet medical need. It can be granted at any point after the FDA approves an IND.

Phase I

If the FDA approves the IND, the experimental drug then moves into Phase I human testing. In this phase, the drug is tested in a small number (under 100) of healthy participants. Researchers look to see how well the drug is tolerated, how it is processed by the human body, and the correct dosing. This process takes a year.

Phase II

Once a compound is found to be well tolerated in healthy individuals, it is then tested for effectiveness for a targeted disease in a small number of patients. In this phase 100-300 people are administered the investigational drug to see if it actually works, and to determine its short-term effects. This process takes about two years.

Phase III

Phase III is a large-scale study of the effectiveness and side effects of the drug in a larger population, usually ranging from 1000-3000 patients. If the drug is submitted to the FDA for approval, the FDA will look at the Phase III data to determine if the drug is safe and effective. Aside from testing the drug's viability, the company producing the drug also determines the logistics involved in creating a large supply of the treatment. Phase III of the FDA approval process takes about three years.

New Drug Application (NDA)/ Biologics License Application (BLA)

If the drug proves to be safe and effective, the company then files an **NDA** or **BLA** with the FDA. NDAs and BLAs are typically 100,000 pages long and include results of human and animal trials as well as information on how the drug is manufactured. It usually takes the FDA 1-2 years to complete the review process and approve a drug. However, there are cases when approval can be accelerated.

- At the time of application **Priority Review** can be granted to drugs that treat an unmet medical need.
- **Orphan Drug Status** is granted to drugs that treat rare diseases, or diseases that have no other available treatments.

Phase IV

Once a drug has received FDA approval it is then marketed to the general population. Short- and long-term side effects continue to be monitored and results are submitted to the FDA. Companies will also look for **additional indication** for the drug. In order for the drug to be approved for a new indication, it must receive approval from the FDA.

Topics: R & D

Which Big Biotechs are hitting the gas pedal on R&D spending?

May 8, 2013 | By John Carroll

The R&D numbers for the top 10 biotechs may only amount to a fraction of what you'll find in Big Pharma. But unlike the giants, which are trying to keep a lid on multibillion-dollar budgets, you'll find a much faster crowd when you turn your gaze to the biotechs. All 10 reported increases in their research spending for last year. And a few of them slammed their foot on the gas pedal.

Altogether the top 10 biotechs spent $11.8 billion on R&D in 2012, according to our research, a hefty 15% average increase over their 2011 performance. Compare that to the stable year-over-year record in Big Pharma, where doing more with the same amount of cash has become an industry mantra.

At Gilead ($GILD), the HIV powerhouse pushed R&D spending up by 43% as it pursued one of the most ambitious late-stage programs in the industry for hepatitis C. The increased spending also pushed them to the number-two spot on the list and left them in the clear lead with one of the most promising experimental therapies in the field. Further, it helped position the company as an emerging player in hep C, ready to blow past the likes of Vertex ($VRTX) and Merck ($MRK) while maintaining its lead over AbbVie ($ABBV), Bristol-Myers Squibb ($BMY) and others.

Even Amgen ($AMGN), the original Big Biotech, upped spending in 2012, despite a decision to trim research costs toward the end of 2011 as its CEO and research chief were being led to the exits.

Overall, the numbers paint a clear picture of a better focused, more nimble and relatively more successful development record for Big Biotech when compared to the average Big Pharma company.

Biogen's record spending, for example, left the company with the hottest new drug for multiple sclerosis, Tecfidera. Celgene ($CELG) has become a partner of choice in the industry with the welcome sight of its checkbook opening doors around the world. Shire ($SHPG) has become a case study in successful growth, with a new CEO putting his own structure and teams in place. Vertex may be about to bid farewell to its hep C franchise, but the company has managed to fill the void with a top-rate combo for cystic fibrosis. And there's another, next-gen hep C effort in the clinic. Regeneron ($REGN) has had perhaps the most successful year in the industry, with one new drug now entering blockbuster territory and some more research programs grabbing analysts' attention. Actelion persevered with a new pulmonary arterial hypertension (PAH) program that emerged a winner in Phase III, while Onyx ($ONXX) has had a winning streak in cancer drug development and BioMarin ($BMRN) expanded with some carefully tailored acquisitions.

New research has also indicated that it's the Big Biotechs which are seeing the most dynamic growth in product revenue as Big Pharma endures a bitter era of generic competition for some traditional blockbusters.

So while the numbers may be smaller, these companies proved to be better focused than most Big Pharmas on key assets. It's the kind of record that can make a biotech a top takeover prospect. It also makes them prime candidates for new deals of their own. -- John Carroll, Editor-in-Chief.

Read more: www.fiercebiotech.com

"*One way to approach investment opportunities in the biotech sector is not by chasing the big names, but by carefully researching second-tier players.*
-- David Miller, BSR's CEO, in Chapter 5 - "The Keys to Biotech Investing"

Investing is by no means simple. In order to become better, time has to be invested. Not all people find the time to research companies, but there are many shortcuts and methods of finding information.

I understand many don't have the time or interest to perform the research themselves, but to those who are, here are some points that I follow.

- Read the documents on the SEC website. It is a mountain of priceless filing information for every publicly traded company.
- Listen to conference earnings calls from earnings.com. The Q&A section is the juicy part.
- Read a few years worth of annual reports.
- Read the competitors annual reports.
- Don't buy because you think you will miss out if you don't buy at the current price. 50% price drops can happen in a day, but sudden 50% rises rarely occur. Don't let your anxiety of missing out tempt you into making bad decisions.

1. Three Stock Valuation Methods

"Price is what you pay, value is what you get." (Warran Buffett)

In other words, price determines value. It's important to know what price to pay based on what the valuation is.

- Discounted Cash Flow
- Modernized Benjamin Graham Formula
- Earnings Power Value

Always with a healthy margin of safety (MOS).

2. Tangible Book Value and NNWC

Tangible book value is a great way to view the asset value of the company at it's face value. A share price made up of a lot of tangible assets will provide downside protection. Intangibles are never easy to value especially if it consists of patents, goodwill and other intellectual property.

The Use of Checklists

I recently came across an interesting article in The New Yorker magazine called *The Checklist* written by a multi-talented surgeon who is also the author of an interesting book I am reading called *"Complications: A Surgeon's Notes on an Imperfect Science"*.

The article is quite long but it boils down to that in spite of strong evidence to the contrary, highly trained people think it's below them to use check-lists as they know what to do and working through a check-list is an insult to them.

From the article:

But this time he found few takers. There were various reasons. Some physicians were offended by the suggestion that they needed check-lists. Others had legitimate doubts about Pronovost's evidence.

This was in spite of these findings:
Within the first three months of the project, the infection rate in Michigan's I.C.U.s decreased by sixty-six per cent.
The typical I.C.U.—including the ones at Sinai-Grace Hospital—cut its quarterly infection rate to zero.
Michigan's infection rates fell so low that its average I.C.U. outperformed ninety per cent of I.C.U.s nationwide.
In the Keystone Initiative's first eighteen months, the hospitals saved an estimated hundred and seventy-five million dollars in costs and more than fifteen hundred lives.
The successes have been sustained for almost four years, all because of a stupid little check-list.

All this from a checklist with steps as simple as "wash hands with soap".

Check-lists work best in a complex environment where the performing of certain steps is critical. In flying it is taken as a given that highly trained pilots work through check-list for virtually every eventuality.

An aeroplane is a complex entity, so is medical procedures and I want to argue so is investing.

Emotional Check

- Write down how you are feeling
- Beware of
  - wanting to just buy and study later
  - hindsight bias
  - overconfidence
  - obligation to buy due to amount of research
  - reluctance to accept differing opinions
  - social proof bias
- If required, take a break and clear your mind. Get away from the excitement and noise.

Final Evaluation

- What can go wrong?
- What are the risks? How likely are the risks?
- How can you lose money?
- How would you categorise this investment?
- How attractive is this idea compared to the other holdings? (There can only be ONE best idea. Not 2 or 3.)
- What is the expected holding time frame?
- What should be the portfolio sizing?
- What price will you sell?

Remember:

When evaluating a company there are so many factors that are beyond your control.

You however, through empirical research, know what increases the probability of you making profitable investment decisions.

What is thus important is that you focus on what you can control in your research and analysis.

Do you need more help on your research process and/or due diligence, concepts, models or tools and checklists, valuation metrics and analysis?

Just contact us, we can help.

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