Young & Partners and Pharmaceutical Executive Magazine

Conference Summary

Pharmaceutical Executive Summit: Emerging Strategic and Financial Issues in the Pharmaceutical Industry - November 12, 2014

The Yale Club at 50 Vanderbilt Avenue - New York City

12:00 p.m. **Luncheon and Welcoming Comments**
Peter Young, President and Managing Director, *Young & Partners*
William Looney, Editor-in-Chief, *Pharmaceutical Executive*

12:30 p.m. **Luncheon Keynote Speaker and Fireside Chat**

Which Pharma Business Models Will Succeed?
J. Michael Pearson, Chairman and CEO, *Valeant Pharmaceuticals*
A Fireside Chat with Michael Pearson
Peter Young, President and Managing Director, *Young & Partners*
J. Michael Pearson, Chairman and CEO, *Valeant Pharmaceuticals*

1:30 p.m. **Challenges and Opportunities in the Pharma and Biotech M&A Market**
Peter Young, President and Managing Director, *Young & Partners*

2:00 p.m. **The FDA Perspective on Personalized Medicine**
Dr. Michael Pacanowski, Associate Director for Genomics and Targeted Therapy
Office of Clinical Pharmacology, Center for Drug Evaluation and Research, *The FDA*

2:30 p.m. **The Pharmaceutical Market: Trends, Issues and Outlook**
Doug Long, Vice President, *IMS Health Inc*

3:15 p.m. **Coffee Break**

3:30 p.m. **Strategy Case Studies: UCB's Transformation Into A Biopharma Company**
Roch Doliveux, Chairman and CEO, *UCB*

4:00 p.m. **Biotech's Business and Funding Fundamentals: What Will the Future Bring?**
Randolph Guggenheimer III, Managing Director, *Young & Partners*

4:30 p.m. **Speaker Roundtable**
Moderator: Peter Young, President and Managing Director, *Young & Partners*
Selected Topics: The Aftermath of the Affordable Care Act; Alternative Pharma and Biotech Strategies; What to do about Pricing?; Innovative Approaches to R&D; Alternative Approaches to Emerging Markets; The Future of Orphan Drugs, etc.

5:30 p.m. **Cocktail Reception – Trumbull Room**

A Fireside Chat with Michael Pearson – A Brief Overview

PETER YOUNG,
President and Managing Director, Young & Partners

J. MICHAEL PEARSON
Chairman of the Board and Chief Executive Officer, Valeant Pharmaceuticals

The fireside chat between Michael Pearson and Peter Young involved a discussion of topics ranging from Mr. Pearson's experience as an undergraduate student at Duke University and his transition after a 23 year career at McKinsey & Co. to pharmaceutical industry management, to Mr. Pearson's thoughts on achieving shareholder and business value in the pharmaceutical industry.

A selection of points made during the discussion includes:

- Mr. Pearson, a native of Canada, was the beneficiary of a great intellectual opportunity in studying math and engineering at Duke University. It was at Duke where Mr. Pearson was exposed to myriad of new things, including college basketball. It is also where he met his wife. He recounted a time, as a student that he had the good fortune to eat a dinner with Steve Jobs and Steve Wozniak, an opportunity he won via a student auction he had entered. He described this as a memorable experience that made a lasting impression on him.
- Michael did not find the transition from McKinsey to working in management in the pharmaceutical industry terribly challenging and he attributes that primarily to everything he learned from industry veterans while serving them as a consultant. He feels that the exceptional quality of the people in the pharmaceutical industry enabled him to gain a great deal of knowledge and valuable experience.
- Mr. Pearson feels that a misunderstood aspect of his management philosophy as an executive in the pharmaceutical industry relates to Research and Development (R&D). He does, in fact, strongly believe in R&D and Valeant has a robust set of new products that are being introduced. However, he feels that industry players have, in some cases, drawn too strong a correlation between absolute dollars spent on R&D and company stock price/valuation. He feels that R&D can simply be done less expensively than it is done by many large pharma companies today and sees the biotechnology industry as an excellent example of that.

Challenges and Opportunities in the Pharma and Biotech M&A Market

PETER YOUNG
President and Managing Director, Young & Partners

I want to talk today about the business factors affecting both pharma and biotech companies that are driving the M&A trends we are seeing in the industry. CEOs and senior management teams tend to forge M&A strategies based on what they see in the business environment and the strategies they are pursuing.

There has been a major disruption in the pharmaceutical industry business model that started ten or more years ago. Under the old business model, pharmaceutical companies were able to develop and commercialize drugs in a 6 to 10 year timeframe and take advantage of reasonable patent lives, favorable pricing, and relatively tame competition when compared with today, to produce strong growth and profits. Much has changed that has damaged that business model. The cost of drug development on average has soared to $1.1 billion per drug, the typical time to commercialization is now in excess of 12 years, drug discovery success rates have decreased due to stricter regulatory approval processes, pricing pressure has increased globally, and patents are being circumvented by governments in certain countries. For the generic pharma companies, the decreasing number of mega brands coming off patent has slowed the growth prospects for generic drugs.

Pharma companies have been adjusting their strategies to survive in the new business environment. Strategies range from diversification, moving to be a pure play pharma company, focusing on leadership businesses and exiting weaker ones, pursuing large scale mergers, exiting the industry altogether, geographic expansions, regional consolidation, pursuit of biologics, expansion of generics, movement into vaccines, pursuit of orphan drugs, movement away from primary care indications to specialized areas, cost reduction programs, etc. Generic pharma firms have shifted and are expanding their R&D capabilities to incorporate biosimilars and specialty generics, moved into new business areas, and are rapidly consolidating.

Although many of these business model challenges for pharma companies also apply to the biotech industry, there are some differences. A number of biotech companies have demonstrated a more effective ability to invent new drugs. The growth of orphan drugs and drugs addressing unmet medical needs in specialty areas has been particularly positive for biotech companies. On the financial side, the stock market has rewarded biotech companies for positive clinical data and a number have been able to raise significant capital in IPOs and follow-on offerings. Venture capital funding has been positive as well.

The pharma and biotech industry M&A numbers are a direct result of these business drivers. In pharma, through the third quarter of 2014, 43 deals worth $73.6 billion were completed versus 42 deals worth $39.9 billion during all of 2013. This represents a significant increase in the number and total dollar volume of deals. This is due in part to companies becoming more aggressive with regard to acquisitions as a way to replenish their pipelines, strengthen their leading businesses, and, in some cases, re-domicile in more favorable tax jurisdictions.

In sharp contrast, the M&A numbers in biotech are very modest and have been for most years. In the first three quarters of 2014 there were only 19 biotech M&A deals completed worth $9.1 billion, of which $3.7 billion comes from the acquisition of Idenix by Merck. This compares to 27 deals worth $7.1 billion for 2013. Acquisitions of biotech companies are driven by pharma companies looking to expand their drug pipelines. However, high stock market valuations and the bidding up of prices for late stage biotech companies have acted as a constraint. The use of partnering and licensing transactions between pharma and biotech companies has also siphoned dollars

away from the M&A market. In addition, biotech companies have heavily exercised the IPO alternative over the past two years as a source of funding and as a route to shareholder liquidity.
Looking forward, what does the M&A future look like for the pharma and biotech industries?

Pharma activity has been escalating, driven by industry structural changes and the strategic moves of the pharma companies. We believe there will be strong volume ahead, even if one adjusts for those deals that were heavily driven by tax inversion benefits. This surge will drive an increase in the fourth quarter and spill over into the first part of 2015. This will continue to be driven by pharma companies modifying business portfolios to focus on leading positions, exiting non-core and mature products, seeking to grow in the emerging markets, and attempting to replace lost or soon to be lost revenues. However, the need to fill the shrinking drug pipeline is also fueling in-licensing arrangements and partnerships and joint ventures involving both pharma and biotech companies, which are not reflected in the M&A data.

Biotech M&A will continue to be active, with the primary theme being pharma and big biotech acquisitions of biotech companies for pipeline enhancement. However, M&A dollar volume will continue to be modest, as pharma and big biotech companies continue to use non-M&A methods to achieve their pipeline goals, such as licensing and partnering and biotech IPOs provide biotech companies with a funding and liquidity alternative. Recent softness in the IPO market raises the question how long the biotech IPO window will remain open going forward.

What are the implications for senior management? For ethical pharma companies, there will continue to be a wide variety of tools to acquire revenues and pipeline drugs, but the valuations are challenging, particularly for promising drugs in late stage clinical trials and for companies with strong products. The challenge will be to pick the right overall mix of M&A, in licensing and partnering that will accomplish corporate strategic goals and defend and deliver shareholder value. The generic pharma companies will continue to face a number of industry challenges. This will result in a continuation of the current generic pharma industry consolidation and selective strategies around diversification. As is true for ethical pharma companies, the challenge of high valuations makes business pivoting by the generics through acquisitions difficult. For all of pharma, the tax inversion route will, in the end, be best used by companies already domiciled in low tax countries.

For the biotech companies finding the right mix of M&A, licensing and partnering channels, coupled with IPO funding and liquidity when it is attractive, will be the key to success. With the exception of the uncertainty around the IPO market, all of these factors will continue to favor the biotech companies. The right answer, however, must be tailored for the circumstances of each biotech company.

The FDA Perspective on Personalized Medicine

MICHAEL A. PACANOWSKI, PHARM. D., M.P.H.
Associate Director for Genomics and Targeted Therapy
Office of Clinical Pharmacology, Office of Translational Sciences
Center for Drug Evaluation and Research, U.S. FDA

I will start out today by giving a snapshot of the personalized medicine activities at the FDA. The key messages I hope you will take away from this discussion include the fact that targeted drug development is feasible, that building a strong scientific foundation first will lead to drugs later on, that data should drive the case for targeted strategies, and that as drug developers and regulators we must all mind the future landscape of the drug development industry in terms of technological changes. Ten years ago the genome was sequenced and today we can sequence a genome in a few days. My personal vision is that in the near future each person's genomic sequence will be identified and be a part of his or her medical record. This will allow for mechanistic approaches to experimental medical therapies supported by pharmacology and real-time synthesis of effectiveness and safety data in the broader healthcare environment.

The current state of drug development is relatively straightforward. Biopharmaceutical companies develop biomarkers in the context of their drug development programs and, either with in-house or external diagnostic professionals, develop tests for those biomarkers with the ultimate goal to bring a drug and an associated test to the clinic. We are however seeing a shift away from the single drug, single test paradigm. In oncology drug development, for example, some clinical trials have implemented genomic profiling. So the true vision of personalized medicine through targeted therapeutics is beginning to show real potential. To fully realize this vision, however, we need to continue to leverage our understanding of biology, do more high through-put exploration and development of biomarkers in early-phase trials, utilize totality of evidence approaches to evaluate risk/benefit in small patient populations, and implement genetic testing technologies in the clinic.

For the rest of my speech I will outline a brief retrospective on what has happened in the past 10 years in the study of genomics, go on to talk about different targeted drug development strategies, and finish by outlining a few opportunities I see for the industry going forward.

It has been just over a decade since the agency truly committed to pharmacogenomics and personalized medicine. Early on, the FDA convened workshops to discuss ways to engage industry participants in order to educate FDA professionals on new technologies being used in developing targeted drugs and to give guidance on how the FDA might handle certain things. Through workshops like this we initiated the "Safe Harbor Concept" which later became the "Voluntary Exploratory Data Submission" program. Both of these initiatives were very successful in bringing together industry and FDA professionals to work through the scientific challenges associated with personalized medicine. Other initiatives that have advanced the mission of targeted therapeutics include the biomarker qualification program, industry investment in biomarkers via PDUFA V, and, ultimately, co-approvals of drugs and companion diagnostics by the FDA.

Some of the things we have learned from recent co-approvals of drugs with companion diagnostics include the fact that evidentiary standards for retrofit biomarkers are not clear and that incomplete sampling has been a problem. We feel strongly that a reliable test is needed to interpret trial results and to identify the to-be-treated population. We have come to stress the fact that if a drug is effective overall, a companion diagnostic may not be

necessary. The FDA has also learned that trials in "marker-negative" patients may be performed post-marketing and that labeling often depends on the studied population as rare subsets exist among "marker-positive" patients. We have taken this knowledge and applied it to our work in regulatory policy and guidance.
Moving on to targeted development approaches, one should first take a look at drug approvals and failures today. If one thinks about the success rate of drugs in phase 2 trials, one can note that 32% of drugs in phase 2 trials achieve success and yet about half of those drugs are ultimately approved. I think it would be nice to see a higher correlation between the success rates of drugs in phase 2 trials with the rate at which those drugs are ultimately approved for use. The most common reason for failure among drug candidates relates to a drug's inability to demonstrate differentiation in efficacy or to demonstrate efficacy in general. This is a problem because it means our drugs are either not doing what they are supposed to or we are selecting inappropriate patient populations. The introduction of personalized medicine should, we hope, solve some of these issues.

If through our understanding of biology and collaborations between both drug and diagnostics developers we can be more focused in our targeted treatments and select the right patient populations, we should be able to either get drugs approved faster and/or kill unsuccessful drugs more quickly. To that end, the role of biomarkers in clinical trial design has become extremely important. Some biomarkers predict response to therapies, some are used to diagnose disease, and some tell you if you are susceptible to disease. Utilizing them in clinical trial work can lead to positive outcomes such as selecting the appropriate patient population or a better ability to select the right dosing schedules or to monitor a patient's response to a drug.

In closing, I want to highlight some of the opportunities with regard to personalized medicine for both industry leaders and the agency. I think we have the tools today to really enhance regulatory science and expedite drug development through improving communication between and within the various relevant offices and divisions of the FDA and drug makers during drug development. I believe that the use of biomarkers and a better understanding of pharmacogenomics will serve to progress this mission. The FDA has placed a focus on advising companies who are focused on diseases with a very strong genetic underpinning who are not evaluating the genetics of the disease to collect data so that the agency has some way to understand whether the drug varies in responses across subgroups.

In closing, we are seeing an intermingling of many forces in the drug development industry and 5 to 10 years down the line I believe that people will have their DNA sequenced and that information will be available in their medical records. If that is the case, applying advanced technology and research, rational development programs and reliable next-generation tests should lead to better patient outcomes.

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The Pharmaceutical Market: Trends, Issues and Outlook

DOUGLAS M. LONG
Vice President, IMS Health, Inc.

I'd like to start out today by highlighting some of the things I find most notable in the pharmaceutical market thus far through 2014. The most notable item here is that double digit U.S. pharmaceutical growth is back and I will explain the reasons why as we go along. Prescription drug growth will finish the year at about 2% which is an improvement from the last couple of years. We have seen many big drugs go generic and OTC recently, and we have seen some generic price inflation as well. Recent aggressive moves by the DEA to police the use of controlled substances have reduced prescriptions in that space. In addition, healthcare coverage sign-ups associated with the ACA are down and projections have been reduced. A few other things to note include new generic labeling rules, the increase in tax inversion deals, and litigation on biosimilars has begun.

Taking a more in depth look at some of this you will see that dollar sales of pharmaceutical products in the U.S. are up 13% YTD and, as I mentioned before, dispensed prescriptions will end the year up about 2%. Some of this growth is due to the rough winter and strong flu season which contributed to sales and prescriptions in the 4th quarter of 2013 and 1st quarter of this year. However we have also now seen 7 consecutive quarters of increased spending on U.S. pharmaceuticals. We have had double digit U.S. prescription spending growth in the past, but in the late 90s it was due to blockbuster primary care product approvals. Much of the growth in U.S. sales are now the result of innovation or new brands and price increases. Many of these new brands are orphan drugs. In fact, there were 17 launches of new orphan drugs in 2013, which was almost half of the total NMEs launched in 2013. Drugs with an orphan indication now make up about 7% of the volume of drugs sold in the U.S. and about 25% of the sales dollars. Some of the stronger recent product launches have included drugs like Eylea, Incivek, Tecfidera, and, of course, Sovaldi.

I would like to move on to talk in more detail about drug prices and, specifically generic drug prices. Whereas there was generic price deflation from 2006-2008, we have seen price inflation since and most significantly in the last year. The reasons for generic price inflation include things such as regulatory quality, customer consolidation and fewer new product launches. With increased scrutiny from the FDA, manufacturers need to invest more in quality systems and when quality/supply issues arise it creates the opportunity to increase prices to recoup part of their investment. With the increased purchasing power of customers, manufacturers need to make up for lost value on products where they can. In addition, generic manufacturers make money by launching new products, reducing cost of goods sold, through M&A activity, and by raising prices. With fewer new launches there is more pressure on the "in-line" product portfolio which is another driver of price increases.

I want to focus now specifically on specialty drugs. What is a specialty drug? A specialty drug is one that treats a specific, complex, chronic disease. These drugs are prescribed only by a specialist, they are expensive, they require reimbursement assistance, and they require special handling and unique distribution. The specialty drug market is now at $106 billion in sales and the growth rate of the industry segment is almost 19%. Specialty drugs now account for about 1% of prescriptions dispensed, but over 30% of all drug spending. Some of the largest specialty markets by sales include oncology, autoimmune, HIV antivirals, and MS. In terms of individual products, some of the largest by sales include Humira, Enbrel, Sovaldi, and Remicade. The top specialty firms by sales include Amgen, Genentech, Gilead, and Johnson & Johnson. To wrap up on specialty drugs, the following are specialty drug related events to watch out for: Sovaldi and orphan drug price discussions, the patient as a payer and specialty tiers in exchanges, growing demand for value driven metrics (CE and RWE)/adoption of guidelines, the crowded specialty space getting more crowded with new entrants and more orals coming, the

first biosimilar application filings, cooperation by payers on co-pay programs, possible 340B changes, and the introduction of gene therapies.

I will now briefly touch on generics. Generic drugs now make up about 82% of U.S. prescriptions dispensed and that is growing, albeit more slowly than in some recent years. The patent cliff has, for the most part, ocurred with the patent expirations in 2012/2013. However there is still a little more pain on the horizon and the worst of it will show up in 2015/2016 and 2018/2019. Some of the major drugs going off patent in the near future include Abilify, Crestor, Cialis, and Lyrica. Some interesting generic drug events to watch out for include: generic price inflation, how generic labeling rules will play out, the upcoming arrival of generic Diovan, Copaxone and Nexium, FDA guidance on biosimilars, Rx to OTC switches (Nexium), the impact of consolidated global generic purchasing, and the expection that there will be more mergers.

In closing, I would like to make some broader points about the pharmaceutical industry and then provide 5 things to watch for. One of the biggest lost opportunities for pharma companies and also the biggest avoidable healthcare cost for the broader system is related to nonadherence by patients who begin a prescription drug regimen. Other large avoidable costs to the healthcare system include delayed evidence-based treatment practice or finding out about a condition too late, antibiotic misuse, medication errors, and suboptimal generics use. The five thoughts I will leave you with include Sovaldi and the Hep C market, generic price inflation, tax inversions, biosimilar applications are in, and continued regulation and reimbursement issues.

Strategy Case Studies: UCB's Transformation Into A Biopharma Company

ROCH DOLIVEUX
Chairman and CEO, UCB

I am going to pass the helm at the end of the year after 10 years at UCB and it is a good moment to reflect on what has happened at UCB during that time. UCB is indeed the result of mergers and acquisitions and we are now the 25th largest pharma company in the world. We are number two in terms of our late stage pipeline productivity and, as a result, our P/E ratio is higher than many of our peers.

Two thirds of M&A transactions fail and yet we were able to grow the market capitalization of UCB by 3 times during my tenure as the result of M&A. I thought that might be useful for some of you to think about that as I describe the transformation of UCB since I became CEO of the company.

When I joined the company in 2003, UCB was a diversified chemical group and our EBIT at the time was about €450 million. We were about to face patent expiries of our two largest pharma products. We had created Zyrtec and co-marketed the product with Pfizer and we were going to lose the patent for Zyrtec in 2007. At the time Zyrtec was about €405 million of the €450 million of total firm EBIT. We were fairly certain that within about three weeks of the patent expiration that we would lose the vast majority of the €405 million. What is worse is that Keppra, our rising star drug, was going to go off patent in the following year. When these drugs eventually did go off patent, the company, between 2008 and 2010, lost more than €1.3 billion. So as you can see our platform was burning and the question was how do we get back to profitability?

Our plan was to transform the company by creating new products focused on severe diseases. Going after treatments for severe diseases seemed to make a lot of sense because the population was aging and pricing was a lot better for severe diseases as opposed to primary care products. In addition, this strategy would give us the opportunity to compete with the larger players in the industry. We placed a particular emphasis on drugs in neurology and immunology. We stuck with this plan and completed a transformation of the company by the end of 2007. To give you an order of magnitude of the change we went through as a company, of the 12,500 employees that were at the company when I joined, only 1,000 are still at the firm that now has a total of 9,000 employees.

Much of this transformation was executed through M&A. In 2004 UCB acquired the UK's largest biotechnology firm, Celltech. Celltech was the first company to discover an Anti-TNF drug and the first to use this type of drug on a human. Today there are 3 Anti-TNF drugs in the list of the top 10 drugs by sales. After Celltech, we divested all businesses that were not pharma related. Then, in late 2006, we acquired Schwartz Pharma, which gave us two additional late stage products. At this time, we were number four in epilepsy and today we are number one. We are also the 4th largest Anti-TNF drug seller and the fastest growing company in the space among the companies in the U.S., Europe, and Japan.

After the transformation of the company was completed in 2007, the company began to really execute its strategy and launch new products. Beginning in 2009 and going forward we launched Cimzia, Vimpat, and Neupro and in 2014 year to date, these products make up almost 50% of UCB's total revenues. We also built the 2nd most productive late stage pipeline in the industry. One of the most exciting discoveries in our clinical development program was the discovery of the gene that causes sclerosteosis, which is a disorder characterized by bone overgrowth. The hope is that this discovery will lead to a drug that can improve bone density in certain patient populations. As a result of our success transforming the company, UCB's stock price rose from floating in the

range of €20 to €30 per share from late 2007 until mid-2010 (during the time of significant losses due to patent expiries), to the price range today which is €65 to €75 per share.

In closing, I would just like to reflect back on UCB's transformation over my tenure at the firm and offer some management guidance. The notion of aiming high but still being grounded in reality is very important. I believe it is Oscar Wilde who said, "If you aim for the stars you may fall among the trees, and if you aim for the trees you will fall on the ground". In addition, I would like to stress that success in large part will be a product of having competent, engaged, diverse teams of people working together to achieve a common goal, and when I say team I do not mean strictly people within the firm. In fact, 50% of our basic research is being done in partnership with university teams and with other pharma firms.

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Biotech's Business and Funding Fundamentals: What Will the Future Bring?

RANDY GUGGENHEIMER
Managing Director, Young & Partners

The biotech industry has grown significantly over the past 30 years and a number of biotech companies have built substantial enterprises. The outlook is positive because of continuing innovation and the relative lack of generic competition for biologics. The growth of orphan drugs and drugs addressing unmet medical needs in specialty areas and the ability to achieve favorable pricing have been very positive for biotech companies. Pharmaceutical companies need new products and product candidates to replace products whose patents are expiring. This has led to strong stock market performance of biotech companies and an outstanding year for IPOs and follow-on offerings in 2013 and 2014 to date. On the negative side, the FDA has been more stringent in demanding proof of safety and efficacy for approval and a number of companies have faced drug safety, manufacturing and reimbursement issues.

Biotech stock price performance has been excellent and financing activity has been extremely strong in 2013 and the first 9 months of 2014. This has resulted in sharply higher P/E ratios for the profitable biotech companies. This has led to massive biotech equity issuance in the first 3 quarters of 2014, including a record number and dollar volume of IPOs. The issuance pace slowed somewhat in the 3rd quarter of 2014, and it remains to be seen if the IPO window will reopen.

The emerging biotech business model of innovative products in specialty and orphan drug markets which allow favorable product pricing has led to strong stock price performance and access to equity financing. The equity financing, in turn, reinforces the ability of biotech companies to build their platforms, remain independent and build significant value in the market.

Agios Pharmaceuticals is an example of this virtuous biotech cycle. Agios was founded in 2007 to discover and develop novel medicines in the emerging field of cancer metabolism. In 2010, Agios entered into a global strategic collaboration with Celgene. In 2011, Agios expanded its research focus to include rare genetic disorders of metabolism. Agios raised $106 million in an IPO at $18 per share in July 2013 and raised $88 million in a follow-on offering at $44 per share in April 2014. Agios' stock price is now over $80 and its market cap exceeds $2.5 billion. These financings allowed Agios to increase its R&D spending by 66%in the first 6 months of 2014. In addition, Agios is now looking to in-license or acquire additional products in cancer and orphan diseases to expand its portfolio.

Intermune is another example of this virtuous biotech cycle. Intermune developed an orphan drug, pirfenidone for idiopathic pulmonary fibrosis ('IPF"). In 2010, the FDA issued a Complete Response Letter and requested an additional clinical trial to support the efficacy of pirfenidone. In 2011, pirfenidone was approved for marketing in the EU. In February 2014, Intermune reported positive top-line data from its Phase 3 ASCEND study and in May 2014, the Company presented the full results of the ASCEND study and resubmitted its NDA. Intermune raised $569 million in convertible debt and equity between January 2013 and March 2014 at prices ranging from $9.90 to $32.75 per share. This allowed Intermune to fund the completion of the ASCEND study and prepare to launch pirfenidone in the US. In July 2014, Roche submitted an offer letter to acquire the Company which led to the acquisition of Intermune at $74 per share in cash, or $8.5 billion. Intermune's market cap had been $808 million as recently as January 2013.

Looking ahead, biotech companies have a bright future as they continue to develop new products, especially for specialty and orphan indications. The equity markets have allowed many companies to raise significant capital and they will be able to pursue independent paths, building even higher stock market values. Pharma and large biotech companies will continue to try to enhance their pipelines by acquiring the most promising biotech companies but M&A prices will be very high for these companies, if they can be acquired at all.

Speaker Roundtable - A Selection of Questions and Answers

Moderator: Peter Young, President and Managing Director, Young & Partners
Participants: Conference Speakers and Guests

Peter Young (Young & Partners): It has been about one year since the original roll-out of the Affordable Care Act (ACA), and we now have access to some results related to the legislation. With regard to the ACA, what do you think is working and not working thus far and what impact has it had on pharma and biotech companies?

Doug Long (IMS Health): I think one positive coming out of the legislation is the potential for healthcare providers to be paid based on treatment of patients as opposed to on a procedure by procedure basis. However it is unclear whether this system will work for healthcare provider systems outside of the larger players such as the Cleveland or Mayo Clinics. On the negative side, the ACA may have had an adverse impact on the price patients are paying for specialist care. Some people may actually be paying more for specialist care than they were under their previous healthcare plans.

Bill Looney (Pharmaceutical Executive Magazine): I think one of the major implications of the ACA is the continued pressure to prove value and demonstrate outcomes through sophisticated use of data. The act has many built-in incentives for healthcare providers and manufacturers of drugs who can provide that data in very structured ways.

Peter Young (Young & Partners): With regard to biotech and pharma companies changing their business models and following a variety of different strategies today, what strategies or business models do you believe are likely to be successful or unsuccessful going forward into the future?

Roch Doliveux (UCB): My bias is to focus. And I realize that it is easier to focus when you are a midsized company than when you are a large company. However, I feel that in the Novartis, GSK, Eli Lilly situations where they are swapping assets to reinforce each other's strategic advantages you are seeing some of the larger firms trying to focus. Also, Novo Nordisk, a midsized company in terms of revenue but large in terms of market cap, has decided to discontinue its R&D related to inflammation and focus on diabetes. So for me, my bias is to be focused and to excel at what you do best. You will see a lot of very successful companies following this strategy.

Michael Pacanowski (The FDA): I cannot speak necessarily on business models broadly, but I can give you a perspective from the regulator side of things. The agency has a vested interest in the success of the companies that are creating therapies that do a lot of important things for patient populations. This is evidenced in a lot of the activity you see in the Breakthrough Program and senior agency leadership involvement in very early phases of drug development.

Peter Young (Young & Partners): What do you think the drivers of successful drug development will be going forward into the future?

Michael Pacanowski (The FDA): I would just say that many of the recent successes have been drugs that have a very large effect on the disease being targeted. In Hepatitis C, a decade ago drugs were achieving 10-15% response rates whereas today they are curing patients. The treatments that have come to market treating 5% of lung cancer patients or 4% of Cystic Fibrosis patients have really done important things for patients and I think this type of focus on solving big issues for patients will be successful going forward.

Randy Guggenheimer (Young & Partners): I would just like to add briefly that a lot of these breakthrough products we have talked about today have an ability to price very attractively and, as such, a lot of companies have really started to develop products with an eye on the pharmacoeconomic environment.

Bill Looney (Pharmaceutical Executive Magazine): I think it is important for drug developers to ensure that their regulatory strategies are fully aligned with their reimbursement strategies because, particularly outside the United States, those previously separated brick walls are now becoming one and the same. Most regulatory agencies outside the U.S. have a mandate to promote access to a therapy in addition to ensuring the therapy is both safe and efficacious.

Peter Young (Young & Partners): I would like to shift gears a little bit and ask, "What is your view of the emerging market landscape, places such as Asia, Latin America, and Africa, and what do you feel are the different strategic approaches that should be taken in these geographic regions?"

Roch Doliveux (UCB): First of all, these are very important markets. Right now some of the growth - although recently we have seen some setbacks - for the large pharma companies are tied to branded generics, although there are a few exceptions and so I continue to push my focused management theory. One example of a company that has been very successful in emerging market countries, and in particular China, is Roche. They have continued to focus on cancer in China and also in Russia and this goes to the point about creating medicines for high unmet need areas and that create real value. Biologics however are, in fact, very difficult to produce and sell in places like China and India.

Doug Long: One thing we have noticed at IMS Health is that in the emerging markets a lot of times the regulatory agency and patient population favor local producers and, at times, the issue for Western pharma companies is market access. The U.S. far and away approves the largest number of prospective products and it is far more difficult to get things approved in places like China or India.

Peter Young (Young & Partners): Do you think that pharmaceutical companies, and particularly those focused on higher priced, orphan drugs, will be able to continue to be successful or do you feel that pushback on drug prices will kill this business model?

Randy Guggenheimer (Young & Partners): I think that the focus of the pricing pushback is really going to be focused on a few select drugs such as the Sovaldis than on the truly orphan drugs for quite a while because the dollars involved, even if they are big to some of the biotech companies, are still relatively small for the payers. Particularly in a situation where a drug is truly saving lives or making a huge positive difference in quality of life, it is very hard for an insurance company to decide not to pay for it. The publicity associated with making that decision is so bad that unless you are talking about a drug selling $8 or $9 billion a year, payers will likely not take that fight on.

Michael Pacanowski (The FDA): I would just add that not all orphan drugs stay orphan drugs. Occasionally these drugs get approved for an orphan indication and then turn out to be effective in treating various different indications. This strategy has been pursued and can, in those types of situations, continue to be successful.