Agenda and Summary of Speeches

Executive Summit: Emerging Strategic and Financial Issues in the Pharmaceutical Industry

November 1, 2017
The Yale Club at 50 Vanderbilt Avenue - New York City

11:30 a.m. **Registration**

12:00 p.m. **Luncheon and Welcoming Comments**
Peter Young, President and Managing Director, *Young & Partners*

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

Innovations and Opportunities in Clinical Development
Stephen Cutler, Chief Executive Officer, *ICON plc*

A Fireside Chat with Stephen Cutler
Peter Young, President and Managing Director, *Young & Partners*
Stephen Cutler, Chief Executive Officer, *ICON plc*

1:30 p.m. **Drug Innovation and the Healthcare Ecosystem**
Ron Cohen, M.D., President and CEO, *Acorda Therapeutics, Inc.*

2:00 p.m. **Challenges and Opportunities in the Pharma and Biotech M&A Market**
Peter Young, President and Managing Director, *Young & Partners*

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

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

3:45 p.m. **Deal Makers Intentions: Where Are We Heading?**
Neel Patel, Managing Director, *Campbell Alliance/inventive Health*

4:15 p.m. **The Strategic Outlook for Pharma and Biotech**
Lisa Henderson, Editor-in-Chief, *Pharmaceutical Executive*
Peter Young, President and Managing Director, *Young & Partners*

4:45 p.m. **Speaker Roundtable**
Moderator: Peter Young, President and Managing Director, *Young & Partners*

Selected Topics: Alternative Pharma and Biotech Strategies; What to do about Pricing?; Innovative Approaches to R&D; The Future of Immunotherapy; Macro Trends Affecting Biopharma

5:30 p.m. **Networking Cocktail Reception**

The Evolving Role of Prescription Benefit Management Companies

DR. STEPHEN CUTLER, Ph.D., MBA
CHIEF EXECUTIVE OFFICER
ICON PLC

ICON runs clinical trials for pharmaceutical companies focusing on programs in Phase One to Phase Four. We've been successfully helping to bring a number of fairly high-profile compounds to market over the last 27 years.

The challenge Pharmaceutical R&D has been facing over the last 20 years has been declining productivity and increasing costs. R&D spending rose rapidly from 1997 to 2007, but has leveled off over the last 10 years. However, the number of compounds that have been brought through development over that time has declined. Development cycles are getting longer. Protocols are getting more complex and are constantly being amended, causing significant delays. There is a huge opportunity to leverage technology and data to alleviate these problems.

One of the challenges we have when we run clinical trials is the lack of participation. Only 3-5% of the population has ever been in a clinical trial. If we could encourage greater participation, it would make a significant difference in the timelines associated with developing drugs and, as a function, the associated costs.

The areas we believe will be crucial to solving the productivity challenges are access to data & analytics, clinical trial design and conduct, new technologies and engaging the patient.

We have a revolution taking place around Electronic Medical Records ("EMR") and around the ability to integrate data from our laboratory, imaging and randomization facilities. This kind of data can be used to identify target patient locations, thus reducing the significant costs associated with recruiting patients.

The sequential manner in which a candidate progresses from Phase 1 to Phase 4 is changing. The Keytruda program ran the largest Phase 1 oncology study ever and got its registration from that study alone. Adapting the dose, inclusion/exclusion, indications as the trial is ongoing, are starting to gain traction, particularly in the oncology space. There are also opportunities to use existing, potentially comparative cohorts of patients such that half of the resources are not spent developing a comparator group. We can better monitor variability and identify outliers by adopting a risk-based approach which examines data as it accumulates.

New technologies include virtual trials, digital patient id, eCOA/PRO, wearables, digitals site management, post marketing surveillance and access to real world evidence. Virtual trials are done directly with patients on a digital platform with limited input from sites. eCOA/PRO involve patients making their own assessments in real time as opposed to intermittent site visits. Wearables monitor almost everything now and transfer data back to a centralized location. Digital site management is helping to encourage investigators to participate in clinical trials by facilitating interface.

As an industry, we have done a relatively poor job of engaging the patient. A number of pharmaceutical companies are starting to seek input from patient groups when designing clinical trials, which allows for more effective enrollment and a better patient experience. Increasing the percentage of the population that participates in clinical trials will have a dramatic effect on their efficiency and our ability to bring costs down.

A Fireside Chat with Steven Miller

DR. STEPHEN CUTLER
CHIEF EXECUTIVE OFFICER
ICON PLC

MODERATOR:
PETER YOUNG
PRESIDENT AND MANAGING DIRECTOR
YOUNG & PARTNERS

Personal

Young: Could you walk us through how your career traced the global path that it did?

Cutler: I grew up in Australia where I did my PhD. After completing that, I realized that I didn't want to spend my life in the lab and I wanted to get into a more practical business-oriented industry. Through an alumnus of my high school, I ended up getting a job at Sandoz monitoring trials. I saw the CRO industry being a larger part of the development landscape and joined Quintiles in the mid 90's. I went back to Australia from the UK and helped them set up a group there then moved to South Africa at a time when Mandela was coming to power. Quintiles took me back to the UK before moving me to the United States in 2003. This is the best place to be working with the top 30 pharmaceutical companies in the world in the tri-state area.

Young: How has your involvement in rugby impacted your career?

Cutler: I grew up playing rugby union in Sydney. I ended up playing for the national team. As such, I'm a competitive sort of person. The CRO industry is very competitive. You win by an inch and lose by an inch. Rugby is a team sport and taught me a lot about recovering from losses, remaining humble when winning and working with people. Rugby has a position for every shape and size and all those positions must come together to score tries. There are no pads in rugby so when you get hit, it hurts. You have to take hits and get up and go again.

Industry

Young: Do you see the relationship between the biopharma companies and their CRO's changing over time?

Cutler: I believe the relationship has improved and CRO's have become more assertive in expressing opinions on trial design and execution to their clients. We constantly struggle with maintaining a balance between servicing the customer and expressing our experienced based views. We need to be better at asserting our point of view in a professional manner. These relationships can survive a little back-and-forth.

Young: How would you characterize the addressable market for CRO's?

Cutler: We still see an opportunity to increase our market. We need to convince our customers that outsourcing is a viable options from an efficiency and cost point of view. Data management is a good example of where our expertise and cost structure are far beyond anything our customers could achieve on their own. R&D budgets continue to inch along so we see some opportunity there.

Young: Do you think that customers benefit by using "one-stop shop" CRO's as opposed to those that specialize?

Cuter: ICON is a "one-stop shop." If customers are looking for "best in class" organizations they need to examine how they are defining "best in class" as well as determine the ability for continuity of that "best in class" distinction. Even then, there is a management component involved with hiring multiple firms creating the potential for oversights. There is an advantage to going with one organization who will put the pieces together.

Drug Innovation and the Healthcare Ecosystem

DR. RON COHEN, M.D.
PRESIDENT & CHIEF EXECUTIVE OFFICER
ACORDA THERAPEUTICS, INC.

The drug industry has borne the brunt of society's discontent about healthcare access and affordable prices. This kind of widespread scorn is not new. Headlines berating the drug industry for pricing have been circulating in cycles for the last 100 years. The downturns in these cycles are usually coincident with a focus on drug pricing and access. Our products are some of the only products on the market that nobody wants to buy. They have to buy them.

Rhetoric against the biopharma industry criticizes its members for being greedy speculators, caring more about profits than patients, spending more on marketing than R&D, and not inventing as many new drugs as the NIH and denying patients access to life-saving medicines.

Antiretroviral therapies, Tyrosine Kinase Inhibitors for Chronic Myeloid Leukemia, Statins and Sovaldi are all examples of drug products that have created significant societal value by reducing healthcare costs where only a small portion of that value has been recouped by drug companies. The biopharma industry returns 14.8% on equity, 45th in a ranking of industries and well below Tobacco and Casinos/Gaming.

As of January 2016, only 8% of publicly traded biotechs were profitable. Only 3% of privately held biotechs were profitable.

In 2014 Industry R&D spend was ~$140 billion compared to ~$70 billion on sales & marketing. Larger companies, often derided by critics, sell in over 100 countries and thus have significantly high marketing expenses. Biopharma has the highest rate of R&D reinvestment of any industry at 18.5% as of 2015.

Private Sector R&D dwarfs NIH Funding by 5 times. Almost 57% of new drugs are developed in the USA as of 2009. Of the 5,293 clinical programs in 2016, 70% were being sponsored by small companies most of which are US based. This is a result of the free-wheeling form of capitalism that is encouraged in the USA as well as a vigorous entrepreneurial culture.

Almost 90% of all prescriptions are for generic drugs. The reduction in the costs of these drugs, as they go off patent, helps to pay for new, more expensive innovations that come to market. However, healthcare costs are still rising systemically primarily driven by hospital and professional costs as opposed to drugs. Third Party Payers/PBM's were pressured the passing of the Obamacare laws to increase prior authorization, step edits and formulary exclusions increasing deductibles, co-pays and "co-insurance costs." The Rebate system incentivizes price hikes. The entire system is geared towards making money off of higher list prices.

Only 1 in 10 compounds reaching the clinic is approved by the FDA. Developing a new medicine takes 10-15 years and over $2.5 billion. These factors make raising money to fund development of these compounds a daunting task. However, in the system we have in the USA there are investors who will take the risk. Adding even a marginal amount of additional risk, whether around pricing or otherwise, may dissuade those investors from taking that risk. As such, the few winners must deliver significant returns on investment to incentivize ongoing investments.

What should Biopharma companies consider changing? The industry has to educate its customers about their products' value vs cost. Direct-to-consumer advertising can play useful roles in disease state awareness and encouraging patient/physician conversations. Our healthcare cost problems are systemic and not just isolated to the biopharma sector.

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

PETER YOUNG
PRESIDENT AND MANAGING DIRECTOR
YOUNG & PARTNERS

Overall Environment

The last few years have been a positive period overall for both the Pharma and the Biotech industries on many fronts. Most importantly, the number of new drugs approved and under development has escalated for both pharma and biotech companies. Many of these are driven by new methods, such as Immuno-Oncology, personalized medicine, stem cells, and biologics. We are also witnessing the development of a greater number of drugs that cure diseases rather than just extend life. The valuations of both pharma and biotech companies in the public and M&A markets soared until the end of 2014, in part because of these positive developments. Negative sentiment related to a host of issues such as the pricing of drugs had an adverse effect on pharma and biotech shares. Share prices and public valuations suffered in 2015 and 2016. Public biotech shares were hit particularly severely after the end of 2014, which ultimately chilled the IPO market starting in the second half of 2015. This is creating a difficult equity financing environment for biotech companies which, in turn, affected the choices available to biotech companies to continue to fund their companies.

Pharma M&A

During the first three quarters of 2017, 18 Pharma M&A deals were completed worth $41.1 billion versus 44 deals completed worth $120.5 billion in 2016. On an annualized basis, this is a dramatic decrease in both the number of transactions and the total dollar volume. In addition, the J&J acquisition of Actelion represented $29 billion of the total, the only mega deal. As of September 30, 2017, the pipeline of deals announced but not closed was also down - a meager $15.3 billion (15 deals). This significant decrease in M&A activity is a reflection of the uncertainties facing the industry and the relative collapse of the specialty pharma companies who drove part of the M&A activity, but not any reduced desire to grow or to enhance business portfolios. There was already a significant drop in M&A volume last year with the loss of the tax inversion driven deals.

Biotech M&A

Biotech M&A activity has almost always been modest historically, with small spurts of activity from time to time. This trend continued in the first three quarters of 2017 with only 18 biotech M&A deals completed worth a mere $3.2 billion. This was a significant slowdown on an annualized basis compared to 2016 when 41 deals worth $20 billion were completed. This was driven by six deals that exceeded $1 billion in value n 2016. This year, the two largest deals in the first three quarters were the acquisitions of CoLucid for $909 million and Ogeda for $535 million. The pipeline of deals announced but not closed as of September 30, 2017 was $12.0 billion (5 deals), a small number of deals, but a larger dollar amount. However, $11.2 billion of that amount was the pending acquisition of Kite Pharma by Gilead.

The Future

The pace of M&A activity through the first three quarters of 2017 has been significantly below last year's pace, which in turn was a drop from the year before. The shutdown of the large inversion deals has been the biggest contributor to the dollar slowdown. Yet uncertainties around other issues such as pricing, attempts to change Obamacare in the U.S., and the turmoil in the specialty pharma sector have all contributed to the slower activity. Still, volume will be likely hit $60 billion by the end of this year, which is not an insignificant number. This activity is driven by the consolidation, restructuring and strategic needs of the pharma companies. Pharma companies are modifying their business portfolios to focus on leading positions and exiting weaker positions, exiting non-core and mature/established products, searching for growth, attempting to replace lost or soon to be lost revenues, and driving for scale and cost reduction through consolidation. Outside of M&A, the need to fill the shrinking drug pipeline is driving in-licensing arrangements and the formation of partnerships and joint ventures involving both pharma and biotech companies. Therefore, in spite of the headwinds from the current ruckus about drug pricing, we believe there will be solid pharma M&A volume ahead of us, but the number of mega deals will be curtailed.

The primary biotech M&A theme has been pharma and big biotech acquisitions of biotech companies for pipeline enhancement. But M&A volume has always been modest, in part because pharma companies used partnering and other techniques to get access to promising drugs from biotech. There was only a handful of larger biotech deals in 2014 through 2016 that were take outs of public biotech companies near or past approval for an important drug. For a few years, promising biotech companies were able to go public first and attract significant interest and high prices later. The surge in IPOs gave biotech companies more flexibility as to whether and when they exited via a sale. The strong secondary issuance market also gave companies flexibility. However, the slowdown in IPOs in late 2015 and in 2016 drove many companies to either sell themselves or to raise funds via partnering deals or discounted private placements. The recent partial revival of the IPO market has given a modest lifeline to private biotech companies. For all of these reasons, we expect the biotech M&A market to continue to be modest, except for an occasional take-out of a post drug approval biotech in a heavily favored part of the industry.

The current explosion of innovation in the drug industry will be a positive overall factor for years to come. However, time will tell how much impact the less positive regulatory, geopolitical, and pricing issues will have. 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 to 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 industry consolidation and selective strategies around diversification. The specialty pharma strategy of orphan drugs and acquisitions appears to be impaired, so a shift in strategies will be needed. For the biotech companies finding the right strategy and the best mix of M&A, licensing and partnering channels, coupled with IPO funding and liquidity when it is attractive will be the key to success.

The Pharmaceutical Market: Trends, Issues and Outlook

DOUGLAS M. LONG
VICE PRESIDENT, INDUSTRY RELATIONS
QUINTILES IMS HOLDINGS, INC.

Sales growth this year has been anemic at low singles digits in contrast to last year which saw ~5% and the year before which saw double digit growth. The potential market entry of Amazon is causing a lot of trepidation, as evidenced by declining market values of retailers and wholesalers. Direct and indirect remuneration fees have put the Pharmacy Benefit Managers in the hot seat. A 40 mg Copaxone generic finally launched. Opioids have been recognized as a national crisis.

Medicines are expected to account for ~14% of healthcare spending. Lipid Regulators, Viral Hepatitis and Anti-Ulcerants are among the slowest growing therapy areas while Autoimmune, Oncology and Diabetes are among the fastest growing.

Spending on traditional medicines is declining while specialty medicine spending continues to grow. Generics are seeing significant price deflation. Net prices are increasing at a slower rate than invoice prices. While unbranded generics prescriptions grew by 1.2%, sales dollars decreased by 9.2%. Patients 50 years of age and over account for 70% of dispensed prescriptions and the majority of growth since 2011.

The pharmacy market is not one that lends itself well to online penetration as opposed to other markets that would likely prove more attractive to a disruptor like Amazon. If Amazon does enter the space, it is likely to be in the cash marketplace where it can use its fulfillment capabilities to supply low-cost generics.

Unbranded Generics capture 85.7% of scripts while accounting for ~13.3% of the spend. 85% of the top 20 generic companies had negative dollar growth over the last year. Three generic purchasers have 75% share of generic purchases. Small Molecule brands that are facing loss of exclusivity in the next 5 years are valued at $72.4 billion.

Regulatory restriction by the FDA and other government entities are resulting in less dispensed narcotics over time. Overdose deaths from heroin and synthetic opioids have grown rapidly since 2013.

Medication adherence is the core of the healthcare value chain. Adherence is influenced by various sociodemographic risk factors including income level, ethnicity, race, education levels, size of household and home value.

Stricter payer management, increased patient out-of-pocket costs, the backlash against drug pricing, and the increasing use of value based models are likely to be more of the same. A shift away from deep rebate model, increased price transparency and increased management within the medical benefit are likely to be game changers.

Deal Makers Intentions: Where Are We Heading?

NEEL PATEL
MANAGING DIRECTOR, COMMERCIAL STRATEGY & PLANNING
INVENTIV HEALTH CONSULTING, INC.

inVentiv Health has been doing a survey for the last nine years specifically around deal making. We interview CEOs, Heads of Corporate Development, Heads of Strategy and other professionals in the space on where they anticipate activity for both sellers and buyers.

It is likely that 2017 deal-making volume will end above historical norm, but below the 2015 record breaking year. It is still a sellers' market with multiple financing options available to buyers along with high buyer demand. A demand surplus exists in CNS (neurology), Hepatic, Hematological and Women's Health indications, while the hottest areas for licensing have remained in immuno-oncology and genetics.

Emerging companies have increasingly greater options in deal-making through the emergence of new buyers and growth in financing. The spread between the % of M&A/partnering deal value with large cap biopharma buyers and financing to small-cap and private biopharma has narrowed quite significantly over the last 8 years. This has allowed smaller companies the ability to retain their assets for longer periods of time and create more value for their shareholders.

Internal factors are a greater driver of deal-making that external factors. Pricing is surprisingly expected to have a relatively limited impact on deal-making. The one external factor expected to play a larger role is the number of FDA approvals. From a buyer's perspective, the benefit of waiting for a product to be de-risked outweighs the incremental price required to acquire those assets.

A greater percentage of buyers anticipate more outright acquisitions compared to sellers. Buyers are particularly bullish on more deals with earn-out components. Buyers are also anticipating fewer IPO's.

Buyers have a significant higher interest in acquiring Phase 3 and marketed assets than sellers are in parting with these late-stage assets. However, sellers are significantly more interested in selling their preclinical assets than buyers are in acquiring.

Buyers and sellers share similar interests in what they consider to be key therapeutic areas with the greatest deal potential. Oncology, Autoimmune and CNS are the top three areas. Buyer interest in Infectious Diseases, Oncology, Autoimmune and CNS assets appears to have tapered relative to supply.

Respondents report an average cumulative conversion rate close to 3.9%.

The Strategic Outlook for Pharma and Biotech

LISA HENDERSON
GROUP CONTENT DIRECTOR, APPLIED CLINICAL TRIALS AND PHARMACEUTICAL EXECUTIVE
UBM LIFE SCIENCES

Young: What are we likely to see changing at the FDA?

Henderson: The FDA has been pushing for expedited clinical trials. Depth of Design is becoming the norm rather than an afterthought. The new Commissioner had been adamant about breaking down the barriers between regulators, innovators and patients. He has been a strong proponent of priority designation and orphan drugs.

Young: Do you have any additional observations on where the debate on pricing is headed?

Henderson: It is going to be very problematic, if states start implementing their own pricing laws. Pharmaceutical companies will then have to spend significant resources on state-by-state compliance. The Byzantine Distribution & Reimbursement System is very difficult for companies to navigate.

Young: Are there any specific strategic opportunities or challenges that you would like to comment on?

Henderson: I think CRO's are at a ~70% penetration rate. That means most drug innovators are not doing their own clinical trials but they still have to oversee them. If you are a small company and you hire a CRO, are you going to get the attention you need? I think the CROs need to proactively make sure that small companies are being serviced appropriately.

Speaker Roundtable – A Selection of Questions and Answers

MODERATOR:
PETER YOUNG
PRESIDENT AND MANAGING DIRECTOR
YOUNG & PARTNERS

PARTICIPANTS:
CONFERENCE SPEAKERS AND ATTENDEES

Peter Young (Young & Partners): What major changes are taking place in the Chinese pharmaceutical market?

Helen Chen (LEK): While growth has slowed down to single digits, it is still the largest market that is growing at high single digits. There has been a strong desire by the CFDA to expedite drug registration and bring it closer to the international average for approval time. There have been a number of initiatives to implement payment protocols for rare disease treatments.

Peter Young (Young & Partners): Has there been any shift away from the hospital-based prescription system?

Helen Chen (LEK): 60% of patients are seen in hospitals, even for outpatient treatment. The government is trying to push care down from the larger hospitals to the smaller hospitals and from the smaller hospitals to community clinics. This effort has met with limited success as the public trust remains with the large institutions.

Peter Young (Young & Partners): Can you comment on how the insurance industry has evolved?

Helen Chen (LEK): The Chinese savings rate is in the mid 40% range. The goal of the healthcare reform initiatives that started in 2009 was universal coverage which is now >95%. It is a public payer system with both urban and rural insurance. Urban insurance pays better that rural insurance, but basic coverage is still there. About 1/3 of the spend is still out-of-pocket which, surprisingly, is comparable to the USA. Private health insurance companies are trying to break through but they have not figured out how to price nor how to sell.

Peter Young (Young & Partners): Do you have any advice for companies interested in becoming more involved in the Chinese market?

Helen Chen (LEK): Many companies that have had success in Phase 2 will find many Chinese pharmaceutical companies seeking the China rights for their products. Companies need to determine if they want to give up that commercial opportunity as well as accept the technology risk associated with those kinds of deals.

Ted Marcuccio (Summit Biosciences): What therapeutic areas are Chinese companies interested in investing in?

Helen Chen (LEK): The biosimilar ship has probably sailed at this point. For every biosimilar company in the USA there are probably five Chinese companies developing the same biosimilar natively. The most attractive areas for them are those with a large population of Chinese patients. Some are unable to assess science and thus decide to wait until the products have been de-risked. Others are willing to jump in early and develop the products for the Chinese market. The larger Chinese companies are ready to put their dots on the map and are acquiring small to mid cap assets all around the world.

Peter Young (Young & Partners): However, the Chinese government has clamped down on companies moving money out of China. Additionally, the Committee on Foreign Investment in the United States can block deals for national security issues. The good news is that if the Chinese government views a deal as strategic, they are more lenient on money coming out of the country. Does anyone have any insight into what is happening with the specialty pharma companies?

Rick Hoyt (Mallinckrodt): A lot of the comments made today are very much in line with our thinking at Mallinckrodt. There is a clear lack of recognition of the value that pharmaceutical companies bring to patients and to payers. Controlled substances are getting a lot of press and it is great that the addiction problem is being spotlighted. However it is important to recognize the real source of that addiction.