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

October 14, 2021
In-Person and Virtual Conference from 12 pm EST to 5 pm EST
Yale Club of New York City and Virtual

Agenda

12:00 p.m. **Welcoming Comments**
- **Peter Young**, CEO and President, *Young & Partners*

12:10 p.m. **Luncheon (in person) and Virtual Networking (virtual attendees)**

1:10 p.m. **Keynote Speaker**

How Cutting Edge Science is Driving the Development of New Medical Products
- **Christopher Austin**, CEO and Partner, *Flagship Pioneering*, and previously Director of the *National Center for Advancing Translational Sciences at the NIH*

1:40 p.m. **Fireside Chat – Translating New Science to Commercial Products**
- **Christopher Austin**, CEO and Partner, *Flagship Pioneering*, and previously Director of the *National Center for Advancing Translational Sciences at the NIH*
- Moderator: **Dr. Stephen P. Spielberg**, MD PhD, Senior Adviser, *Young & Partners;* former Deputy Commissioner for Medical Products and Tobacco, *FDA*

2:00 p.m. **Innovation in Clinical Development**
- **Stephen Cutler,** Chief Executive Officer, *ICON plc*

2:30 p.m. **The Pharmaceutical Market: Trends, Issues and Outlook**
- **Doug Long**, Vice President, *IQVIA*

3:30 p.m. **Virtual and In Person Networking Coffee Break**

4:00 p.m. **The Pharma and Biotech M&A and Financing Landscape**
- **Peter Young**, CEO and President, *Young & Partners*

4:30 p.m. **Strategic Challenges Facing the BioPharma Industry**

- **Lisa Henderson**, Editor-in-Chief, *Pharmaceutical Executive*
- **Michael M. Shih,** Head of Business Development, *Biogen*
- **Peter Young**, CEO and President, *Young & Partners*

5:00 p.m. **Conclusion of the Conference**

Young & Partners

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How Cutting Edge Science is Driving the Development of New Medical Products

DR. CHRISTOPHER P. AUSTIN
CEO-PARTNER
FLAGSHIP PIONEERING

During my professional career, the medical community's ability to treat disease has improved dramatically. What was science fiction 35 years ago is now being done by high school students. The vast majority of diseases do not have FDA approved treatments. However, our knowledge of the causes of the diseases, at a molecular level, has improved dramatically. This improvement has come despite the efficiency of therapeutic development declining. The number of new drugs approved by the FDA per billion U.S. dollars spent on research & development has decreased by 50.0% roughly every 9 years since 1950.

Most stakeholders do not understand the complexity of trying to develop a new medical product. The term "pipeline" is very misleading in that it oversimplifies the incredibly complex process that is drug development. The National Academy of Medicine's Forum on Drug Discovery, Development and Translation attempted to create a detailed "traffic map" of how the process works in order to educate stakeholders and identify opportunities for innovation.

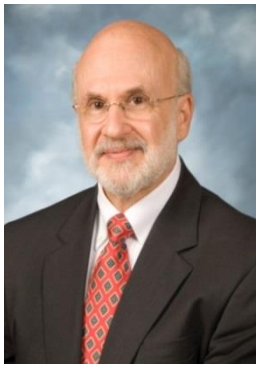
The National Center for Advancing Translational Sciences (NCATS), of which I was the founding Director and part of for many years, was created to bridge the gap between scientific discovery at the academic level and the efficient development of treatments. Most compounds fail in development due to efficacy. This results from the inadequacy of the animal models used to determine the potential for clinical efficacy in humans. This has led to the development of microphysiological system tissue chips that emulate organ physiology. These chips have been adapted to model diseases and predict drug efficacy. There has been a proliferation of companies developing tissue/organ chips.

The holy grail of gene therapy has been the equivalent of spell-check. It will become possible to engineer enzymes that exist in nature to repair individual misspellings. It is analogous to find-and-replace in Microsoft Word. This technology may ultimately allow us to treat many diseases at a time and use common delivery vehicles for therapies and manufacture therapies more efficiently. Challenges can include irreversible changes and off-target effects.

When I moved to Flagship Pioneering, I found that has a lot of similar characteristics to NCATS but the difference is that we are actually developing products. The company applies its science-inspired business model to the portfolio companies it forms. The companies go through four phases: Hypothesis Testing, Feasibility Generation, Internal Venture then Spinout Venture.

Fireside Chat – Translating New Science to Commercial Products

DR. CHRISTOPHER P. AUSTIN
CEO-PARTNER
FLAGSHIP PIONEERING



DR. STEPHEN P. SPIELBERG
SENIOR ADVISOR, YOUNG & PARTNERS
FORMER DEPUTY COMMISSIONER FOR MEDICAL PRODUCTS,
FOOD AND DRUG ADMINISTRATION

Dr. Spielberg: Do you have any thought on how we overcome the communication gap that exists in healthcare between the various stakeholders?

Dr. Austin: I think its really important for healthcare professionals and scientists to stay humble. People have a tendency to forget how little we really know about nature. There is a missplaced sense of absolute certainty. Its difficult to get public health officials, politicians, etc. to adopt that ethos. I think the best way to stay humble is to continue to work with patients. Patients, while generally supportive of scientific research and discovery, cannot be treated with publications.

Innovation in Clinical Development

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

Adoption of innovations supporting clinical operations improvements can take 15-20 years before they achieve widespread adoption. There are still some clinical trials capturing data on paper.

Post pandemic, key industry trends include (1) increased patient centricity (2) technology adoption and (2) scientific advancement acceleration. Patients need to be offered trials that are relevant, convenient and accessible. Decentralized Clinical Trials (DCTs) attempt to remove some of the barriers that prevent patients from participating in research. Technology is also enabling new models that seek to offer Clinical Research as a Care (CARE) option. Scientific advancement is happening at an accelerated pace and the number of next generation therapeutics has doubled.

The shift towards virtualize CARE models with connected devices is fueling development of predictive algorithms that can provide early warning to remote care teams and help to improve outcomes. With the emergence of Digital Therapeutics, we are seeing a convergence of biopharma and medical devices.

We see an increased number of our clients focused on maximizing their investment in clinical research. This can be achieved using tokenization - a way of linking de-identified data sets whilst preserving patient privacy. ICON was the first CRO to tokenize clinical trial data.

Opportunities for decentralization of clinical trials include fit-for-purpose protocols, real time data, and delivery skill set evolutions. Challenges include both regulatory standards and commercial model maturity. Execution of DCTs is complicated and requires thoughtful assembly of all the components – not just technology platforms, but also data flow, drug deliver, chain of custody, etc.

ICON's exclusive SynomaID® provides a HIPAA-compliant methodology for accurately linking patient level data across sources without exposing protected health information (PHI). The technology is used to create detailed patient journeys by linking patient records, including traditional claims and prescriptions data, electronic medical records, lab data, patient registries and genomics data for modeling and analysis.

The application of Machine Learning and Artificial Intelligence to read and understand complex protocol criteria and to match them to concepts in patient data for feasibility and eligibility holds great promise. Through the integration of data sources - laboratory, pathology and electronic medical record (EMR) data, and application of artificial intelligence / natural language programming to the evaluation of patient data, we can match patients to protocol inclusion/exclusion criteria automatically and continuously. ICON is partnering with Deep Lens to demonstrate this approach. Deep Lens is deployed across 172 community oncology sites in the US. The initial feedback is promising and we are working to collect data – evidence of efficacy– to compare recruitment rates across Deep Lens and non-Deep lens sites.

If we can broaden access and awareness of clinical trials, we can potentially recruit more patients at a faster pace, and subsequently accelerate clinical development of new drugs and therapeutics through an efficient, modern process. This modernization benefits sponsors and patients by reaching a more diverse population, whilst exceeding recruitment rates and ultimately speeding time to market.

The Pharmaceutical Market: Trends, Issues and Outlook

DOUGLAS M. LONG
VICE PRESIDENT, INDUSTRY RELATIONS
IQVIA HOLDINGS INC.

COVID-19 Cases and Deaths are trending downwards. The statistics can be misleading as hospitals are incentivized to report deaths from COVID-19 if the patient was infected at time of death. Most of the COVID-19 deaths have been in people >65 years old. 56.2% of the entire US population is vaccinated.

There has been a significant gap between expected diagnostic visits (based on historical levels) and actual diagnostic visits in 2020 and thus far in 2021. Missed diagnosis visits have a direct effect on prescription utilization. Telemedicine has contributed to weak growth in medical claims.

Specialty pharmaceutical growth is outpacing traditional growth. Immunology and Anticoagulants have shown the greatest growth over one- and five-years. Manufacturer net revenues have increased by ~$48.0 billion over the past five years.

Patient adherence to medication is not uniform regionally and varies by different factors such as method of payment. 90-day prescriptions are an effective way to increase adherence rates.

In 2021, 85.9% of prescription are unbranded generics however, unbranded generics accounted for less than ~20.0% of the spend. Savings over the next five years as a result of biosimilars are projected to exceed ~$100.0 billion. The impact of exclusivity losses will increase to ~$166.0 billion over the next 5 years.

The rate of drug overdose deaths is increasing despite declines in prescription opioid use. Most of these deaths are due to Fentanyl.

Multiple types of digital health tools contributed to mitigating the impact of the pandemic and are now established part of the digital health landscape. The COVID-19 pandemic had a significant impact on the apps individuals downloaded and used in 2020, including telemedicine apps such as Doximity, and downloads increased 38x, along with exercise apps that helped patients stay healthy, mental health apps to manage depression, anxiety or suicidal thoughts, and blood pressure apps.

Pharmaceutical companies continue to bring new products to market despite the uncertainty introduced by the COVID-19 pandemic. There were 71 launches through December 2020 in line with 69 launches for the same period in 2019. To date, 2021 has seen a similar rate of product launches compared to 2019 and 2020. COVID has not significantly impacted the number of launches. The largest category of new launches had been Oncology. Novo Nordisk's Wegovy for weight loss leads the 2021 launches in terms of sales, followed by Sanofi's Menquadfi, the first quadrivalent meningococcal vaccine. Gilead's Veklury for COVID-19 leads 2020's launches in sales followed by Horizon's Tepezza (thyroid eye disease) and Allergan's Ubrelvy (acute migraine). Products launching during the pandemic lag pre-COVID launches by ~50% to ~75%, with long-term implications.

Our outlook for 2025 is that U.S. market growth will return to pre-pandemic projections, policy changes impacting the use of medicines are likely to be phased in over the next five years; new brand spending in the U.S. is projected to be higher than the last five years but will be a smaller share of total spending, and immunology, oncology, and neurology will drive growth.

The Pharma and Biotech M&A and Financing Landscape

PETER YOUNG
CEO, PRESIDENT AND MANAGING DIRECTOR
YOUNG & PARTNERS

The current business and financial environment is both positive and negative for the pharma and biotech industry. The high degree of successful innovation by both pharma and biotech companies, the higher number of FDA drug approvals, the regulatory changes that have been favorable to the industry, and the success of orphan drug efforts have all contributed to the industry's success. However, there are an equal number of negative factors, such as pressure on pricing, the stubbornly high cost of drug development, and periodic safety and manufacturing issues that are putting negative pressure on the industry. The biopharma industry's efforts related to the pandemic have been a positive in terms of the public's perception of the industry.

Pharma & Biotech M&A

There were only 9 transactions in the first three quarters of 2021, a significant decrease from the quarterly pace exhibited in recent years. The 9 deals that were completed were worth $48.9 billion versus 25 deals completed in all of 2020 worth $109.1 billion. The relative focus of the large pharma companies has been on acquiring biotech companies and the use of strategic alliances, joint ventures and in licensing to achieve pipeline growth. There has been far less focus on acquiring pharma companies. In terms of backlog, as of September 30, 2021, the dollar value of deals announced but not closed was $26.7 billion (9 deals), the largest of which being Merck's $11.6 billion purchase of Acceleron.
Even with the slight pick-up in volume based on the back-log, it will be difficult to achieve last year's volume by year end.

Biotech M&A activity has been historically modest, with small spurts of activity from time to time. However, that has changed in the last year or so and continues today. In the first three quarters of 2021, activity in terms of the number of deals is already equal to the number of deals in 2020, but has fallen back in terms of dollar volume. 32 deals worth $17.8 billion were completed versus 32 deals worth $52.8 billion completed in 2020. Deals are happening at an earlier stage, which is contributing to the smaller average deal size. The dollar value of the pipeline of deals as of September 30, 2021 was $8.2 billion (9 deals).

Biotech Equity Financing

Biotechnology has fallen in and out of favor with the equity issuance market over the years. In the first three quarters of 2021 public equity financing was at a record pace. In terms of the number of equity financings, volume in the first three quarters of 2021 was also at a record pace. IPO volume in terms of number of IPOs in the first three quarters of 2021has already put the year at a record level. IPO dollar volume in the first three quarters of 2021 has also already exceeded the previous annual record.

Pharma & Biotech Future Outlook

The business outlook for pharma companies is positive with regard to drug development, with many promising drugs in the pipeline. The industry's drug development innovation and productivity has improved after a long period of lower productivity. When combined with the strong contribution of acquired and partnered drugs from the biotech industry, the overall development activity has been strong and will continue to be strong. There has been a shift in emphasis towards orphan drugs, oncology therapies, new innovations such as gene therapy, Car-T, immune system solutions, CRISPR, etc.

The current pandemic has been a plus for the reputation of the industry, but a negative with regard to the obstacles that the pandemic created for those executing clinical trials and related to the maintenance of industry supply chains.

Young & Partners expects pharma M&A activity to be relatively modest in dollars and number of deals in 2021 and that will continue into next year. Most of the market focus will be on medium sized deals and the number of deals will be less. Deals with a strong strategic rationale or a theme around adding new and growing technologies and products will continue to be pursued. Mega deals that merge existing mature bio pharma companies are no longer being pursued. More of the M&A will continue to be directed towards the acquisition of biotech companies. In addition, most of the attention will be on in-licensing arrangements, partnerships and joint ventures with biotech companies and other pharma companies.

The development capabilities of biotech companies have been and will continue to be positive overall. Although there will be successes and failures by individual companies, biotech companies have demonstrated their ability to develop new drugs at a faster pace than the larger pharma companies. M&A volume in terms of numbers of deals will be strong through the end of 2021, but dollar volumes will lag behind last year's total. Buyer interest will be focused on specific targets in favored therapeutic and technology areas and on biotech companies that have made significant clinical progress.

This will be a strong year for biotech company funding, as biotech companies access the equity markets, and pursue partnering, licensing and royalty monetization for funding and for shareholder liquidity. Secondary equity offerings and IPOs will continue at record rates as long as the market continues to favor biotech companies.

Strategic Challenges Facing the BioPharma Industry

MICHAEL SHIH
HEAD OF BUSINESS DEVELOPMENT
BIOGEN INC.

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

PETER YOUNG
CEO, PRESIDENT AND MANAGING DIRECTOR
YOUNG & PARTNERS

Young: What are the potential opportunities and challenges associated with digital patient care?

Shih: The point of digital is to get closer to patients and that is going to drive the suite of offerings made available to the patient. Companies are considering what tools beyond the therapeutic they can offer to enhance the patient experience.

Henderson: There are a lot of touchpoints with patients in clinical trials where digital solutions can be applied. There is a proliferation of vendors purveying "digital health solutions."

Shih: Furthermore, there are regional differences that dictate which digital solution is most appropriate. Another consideration is how clinical data can be used to guide treatment.

Young: Can you discuss how pharmaceutical companies are using patient data in their drug development efforts?

Shih: I know genetics data is being leveraged in translational medicine. There are also regional differences in privacy laws that affect the quality of the data that you can harvest in various parts of the world.

Young: What major challenges do you see the biopharmaceutical industry facing over the next 5-10 years?

Shih: I think there will be debate about the value proposition of gene therapy. There are a lot of questions around the business model. Do you pay for years of outcome? Do you pay per treatment? Additionally, the sheer volume associated with the emergence of the Chinese biopharmaceutical market will force the industry to address its potential.

Henderson: The profile of the workforce in the biopharmaceutical industry, like in so many others, will be increasingly scrutinized. How will biopharmaceutical companies react to the Diversity, Equity and Inclusion push? Furthermore, a lasting impact of the pandemic is the widespread adoption of remote-work policies. Biopharmaceutical firms will need to think about how they will attract and retain talent in an evolving employment market.

Young: Can you elaborate on the unique facets of the Chinese biopharmaceutical market?

Shih: The scale of the market demands scale of investment in order to be effective. On top of that, mandated government pricing requires that return on that investment be recuperated in a relatively short period. It is also hard to keep refilling the pipeline to effectively leverage that scale once it is in place.

Henderson: The infrastructure to support supply chains is still developing in China, as well as, in APAC more broadly.

Shih: It will be interesting to see how the Chinese government responds to domestic innovation. Will they relax regulatory standards to make the domestic market more competitive?

Young: How real of a threat is Chinese innovation to Western biopharmaceutical companies?

Shih: There is a lot of venture money flowing into the Chinese biotechnology industry. There is very little Western biopharmaceutical companies can do other than to make efforts to capitalize on the innovation coming from that part of the world.

Henderson: There is a lot to be said for the innovative spirit that Chinese nationals adopt when working for Western biopharmaceutical companies. It is unclear whether China will ever be able to replicate that domestically.

Young: What are your thoughts on how the relationship between big pharma and clinical-stage companies is evolving?

Shih: I think fundamentally the relationship between the two types of firms has remained symbiotic. I'm curious as to whether the firms that get absorbed into big operations remain innovation hubs. The process of decision-making and budgeting in large organizations is so diffuse, that innovation is not always prioritized. It's rare that the talent acquired chooses to stay in that type of environment. Loxo Oncology seems to have been a rare exception.

Henderson: From my perspective, Pharmaceutical Executive has increased our coverage of clinical-stage companies since I first joined. There are PhD students starting companies immediately after leaving academia. They expect big pharma to ultimately control their technology and rarely do they envision taking their technology beyond a certain stage of development.

Young: Does the biopharmaceutical industry have a role in addressing escalating healthcare costs?

Shih: If you really want to bring down costs, you probably have to start with tort reform. A lot of these costs reflect the potential liabilities of litigiousness. Tort reform would likely bring down insurance costs. Physicians would be less incentivized to order extraneous diagnostics in order to protect themselves.

Henderson: PhRMA has tried to communicate that, as a percentage of overall costs, pharmaceuticals are a small percentage of overall healthcare spend. They have also extolled the healthcare savings that pharmaceuticals create i.e. shorter length of hospital stays, etc.