

PHARMA LANDSCAPE 2020

2013 IQ SYMPOSIUM REPORT

ADDRESSING OPEN INNOVATION, GLOBALIZATION, & PATIENT FOCUSED MEDICINE

The third annual IQ Consortium symposium, “Pharma Landscape in 2020”, was held November 19, 2013 in Alexandria, Virginia. In line with the IQ mission, the event brought participants together to address three key issues influencing the future of the pharmaceutical industry: open innovation, globalization, and patient-focused personalized medicine.

Keynote Address

After two of his children were diagnosed with Pompe’s disease, a rare and fatal neuromuscular disorder, John Crowley founded a company focused on developing a treatment for the disease. Over the course of more than a decade, Mr. Crowley has continued to lead the efforts to develop a treatment for this disease and has experienced firsthand the need for risk taking and innovation within pharmaceutical research.

In the development of treatments, Mr. Crowley remarked that one sells hope and promise tempered with reality and good science. The high chance of failure cannot prevent industry from moving forward. It is impossible to avoid taking risks during research, as most drugs under development will not work or will not work as originally intended. Further, the pharmaceutical industry cannot settle for small, incremental

steps but must attempt to move forward with “swing for the fences” ideas.

Along his journey, Mr. Crowley realized the need for hope, humility, and persistence. Scientists and patients must have hope for progress in order to overcome challenges each day, and must recognize that today’s work is laying the foundation for iterations of success. Humility and perspective have the power to heal, to enhance, and to save lives. Persistence is required to overcome the clinical and business setbacks that are common throughout R&D.

Mr. Crowley noted his experience has been a long process of trying, failing, adjusting course, and trying again. Research is a fight to bring needed treatments to patients, and Mr. Crowley emphasized the importance of maintaining a sense of urgency and fire.



John Crowley is chairman & CEO of Amicus Therapeutics. His story provided the inspiration for the major motion picture, “Extraordinary Measures”.

Interested in More Information?

In addition to an exceptional faculty of speakers and moderators from within and outside of the pharmaceutical industry, the Symposium featured breakout sessions on the symposium subtopics and live audience polling. To watch a highlights video and view the full polling results, visit iqconsortium.org/news/landscape2020.

Report Photo Credits: David Christopher

Open Innovation

Chris Haskell, PhD; Head US Science Hub, Global Drug Discovery, Bayer HealthCare

The pharmaceutical industry is facing significant challenges today, and is increasingly looking to new models of interaction and collaboration, including aspects of open innovation. Dr. Chris Haskell discussed two primary forms of open innovation in his talk, offering examples of each within the pharmaceutical industry.

The first approach to open innovation addresses common challenges in pre-competitive space. Examples include the IQ Consortium, Innovative Medicines Initiative (IMI), TransCelerate, and Enlight Biosciences.

The second approach is through strategic partnerships among stakeholders such as pharmaceutical and biotechnology companies, academia, and others in regions known as “Innovation Ecosystems”. This format is especially common in the area of early discovery.

While many open innovation initiatives are still relatively new and frequently focus on concepts that apply to the early stages of pharmaceutical development, Dr. Haskell emphasized that open innovation can be applied throughout the entire R&D process.

Dr. Christopher Haskell leads Bayer's U.S. Science Hub, based in the company's U.S. Innovation Center located at Mission Bay, San Francisco.

Chris Haskell spoke about open innovation and the importance it has in the R&D process.



Breakout Sessions

This year's symposium featured six breakout sessions - three for each subtopic (Open Innovation, Personalized Medicine, Globalization), with a CMC or Biology perspective. Participants engaged in discussion about the challenges each posed to the pharmaceutical industry, and brainstormed possible solutions IQ could explore.

Open Innovation Breakout Sessions

Chemistry, Manufacturing and Control Breakout

- ◆ **Challenge to Innovation:** The short-term focused societal norm.
- ◆ **Potential Solutions to Facilitate Innovation:** Establish an IQ postdoctoral fellowship program in collaboration with the National Institute of Standards and Technology (NIST) and/or the National Center for Advancing Translational Sciences (NCATS) at NIH, to create an open cloud space for IQ to exchange ideas, knowledge and data, to define and raise awareness of open innovation, to collaborate and drive change proactively.

Biology Breakout

- ◆ **Challenges to Innovation:** Legal barriers and IP concerns, company bureaucracy, budgets, identification of the right individuals to implement ideas, risk-averse culture, and the fear of lowering barriers for competitors.
- ◆ **Consequences if Challenges are Not Addressed:** Slowing of drug development processes, increased costs, and contraction of the industry leading to a reduced ability to address the needs of patients.
- ◆ **Potential Solutions to Facilitate Innovation:** Better define the pre-competitive space, building strong business case for open innovation initiatives, and obtain greater managerial buy in to expedite the decision-making process.



Join the IQ Consortium LinkedIn Group!

We have recently launched our LinkedIn Page. Keep in touch with everyone you met during the symposium. Check the LinkedIn group often to keep aware of IQ accomplishments and events!

Personalized Medicine

Robert Sweetman, MD; US Medical Affairs, Group Lead for Thoracic and Hematologic Malignancies; Pfizer, Inc.

Patient focused personalized medicine is a multi-faceted concept that has become increasingly common in recent years. It is viewed by IQ Consortium participants as an approach that encompasses precision medicine, proactive care, stratified medicine, individualized medicine, and targeted therapy, and it holds promise for individuals facing a variety of diseases. Dr. Sweetman and Richard Heiman's on-stage discussion about Richard's decade-long experience battling cancer brought the concept of personalized medicine to life for IQ Consortium participants. During their dialogue, Richard related

the story of his initial diagnosis with lung cancer, subsequent treatments, remission, and numerous recurrences. He and Dr. Sweetman discussed how enrollment in a clinical trial for Xalkori, a medicine intended to treat a subset of cancer patients with specific genes – a subset that included Richard – offered new hope for Richard, and has significantly prolonged his life. Dr. Sweetman and Richard also discussed the challenges of participation in clinical trials, and emphasized the need for awareness among patients and doctors of the ever-evolving field of personalized medicine.

At Pfizer Oncology, Dr. Sweetman leads the US Medical Affairs team for Thoracic and Hematologic Malignancies. His focus over the last few years has been dedicated to development of Xalkori (crizotinib) which culminated in the accelerated approval by the FDA on 26 August 2011.

Richard Heiman spoke on patient focused medicine.



Personalized Medicine Breakout Sessions

Chemistry, Manufacturing and Control Breakout

- ◆ **Challenges to a Personalized Medicine Approach to Drug Development:** An accelerated timeline, and the need for a shift from high production to smaller scale manufacturing processes.
- ◆ **Consequences if Challenges are Not Addressed:** Validation failures and approval, commercial, and post-approval issues.
- ◆ **Potential Solutions to Facilitate a Personalized Medicine Approach:** Shift in mentality toward defining target product profiles more in line with personalized medicine, development of small-scale predictive modeling tools and use of small scale processes, design of more efficient experiments, development of better methodologies for accelerated production, and sharing of manufacturing capabilities. From a regulatory perspective, more frequent and productive exchange with regulatory agencies and adaptation of regulatory review and approval approaches will be key to the management of these challenges.

Biology Breakout

- ◆ **Challenges to a Personalized Medicine Approach to Drug Development:** Potentially profit margins due to smaller patient populations; insufficient knowledge of certain diseases, phenotypes and the magnitude of benefit from treatment; difficulty in identification of markers and target populations, and complexities in clinical trials.
- ◆ **Consequences if Challenges are Not Addressed:** Costly drug development, treatment resistance, and difficulty in retaining good patient candidates.
- ◆ **Potential Solutions to Facilitate a Personalized Medicine Approach:** Increase understanding of disease mechanisms and biology; consider biomarkers in earlier stages; and develop better animal models, negative predictive biomarkers, and other disease modeling tools. The discussion group proposed the establishment of a Translational Medicine/Science Leadership group, sharing of early development techniques to identify good proof-of-concept study designs, sharing of lessons learned



Want to share your group's achievements?

Want to see your leadership or working group's achievements and successes shared with the rest of IQ? Contact Kathryn.sweigart@dbr.com to have your group's news posted on the Website, in the publications directory, or shared on LinkedIn.

“The industry is moving towards a model that is much more dependent on sharing opportunities and sharing the risks. IQ was created... to facilitate that collaboration.”

Lewis Kinter,
IQ Consortium Chair

Globalization

Globalization and the Cloud – Gabor Fari, Director of Business Development and Strategy, Health and Life Sciences at Microsoft

Mr. Fari opened his presentation stating that globalization is more than just technology and requires cultural changes to be successful. Pharmaceutical companies have often been late adopters of certain new technologies, a sentiment that polling results showed was reflected by a large percentage of audience members.

“The cloud” and its impact on globalization was the main focus of Mr. Fari’s presentation. The cloud allows

for seamless connectivity and sharing of data and computing resources, independent of their location, operating system, or device. The security of data stored in the cloud and moving to/from the cloud is maintained through the use of standards and various encryption technologies. Cloud computing is the new paradigm and will affect the way the pharmaceutical industry conducts business and operations in the future.

Gabor Fari is a founding team member of the Health & Life Sciences Industry Unit at Microsoft, starting in 2006. He has been involved with the biopharmaceutical industry throughout his professional career. Gabor plays a key role in defining and executing Microsoft’s Life Sciences solutions and business strategy.

Globalization Breakout Sessions

Chemistry, Manufacturing and Control Breakout

- ◆ **Challenges Associated with Globalization:** Variance in regulations, customer expectations, manufacturing requirements and IP concerns in different regions. Communication and knowledge transfer globally represent another significant challenge.
- ◆ **Consequences if Challenges are Not Addressed:** A potential loss of competitive advantage – higher costs, product delays, and an increase in quality concerns about the supply chain.
- ◆ **Potential Solutions to Challenges Associated with Globalization:** To address these issues, participants stressed the importance of global harmonization of regulatory requirements as well as formulation and packaging practices. IQ should try to recruit more member companies and individual participants based in other countries, establish more collaborations with worldwide organizations, share best practices in different world regions, and develop strategies for the effective use of the Cloud.

Biology Breakout

- ◆ **Challenges Associated with Globalization:** Increased costs, communication difficulties, variations in government regulation, material import issues, and IP protection concerns.
- ◆ **Consequences if Challenges are Not Addressed:** The industry will miss valuable opportunities to do business and to benefit patients.
- ◆ **Potential Solutions to Challenges Associated with Globalization:** Sponsorship of international educational programs and increased relationship building and collaboration were presented as activities that IQ is uniquely positioned to undertake to enable globalization.

Upcoming Event: IQ GMPs Workshop

“Best Practices and Applications of GMPs for Small Molecule Drugs in Early Development”.

February 4-5, 2014

2014 GMP WORKSHOP

Best Practices and Application of GMPs for
Small Molecule Drugs in Early Development

Recognition Awards

Margaret Faul, Amgen, Inc.

Margaret Faul has been a driving force for the API Leadership Group since it was formed in 2011, and active as an initiator and leader for three Working Groups: the Radiolabelled Materials Working Group; the API Starting Material Working Group and the Enabling Technologies Exploratory Working Group.

Michael Fossler (GSK)

Mohamed El Mouelhi (Novartis)

Drs. Michael Fossler (GSK) and Mohamed El Mouelhi (Novartis) for their exceptional work in initiating and organizing the 2012 industry symposium on regulatory and scientific challenges in pediatric clinical studies. Based on their work, the CPLG is continuing its activities addressing challenges in the pediatrics space, into 2013/2014, with the goal of continuing to engage regulatory as well as industry thought leaders.

Michael Kastello, Sanofi

In 2011, Mike worked closely with Dr. Lew Kinter, to develop the proposal for the creation of the 3Rs Leadership Group of the IQ Consortium. Mike served as the founding chair of our 3Rs LG and was an outstanding leader.

Ingrid Mergelsberg, Merck & Co.

Between 2011 and 2013, Ingrid has dedicated significant effort to leadership of the API Leadership Group as well as IQ's Green Chemistry Working Group. As leader of the Green Chemistry Working Group, Ingrid has helped to initiate important dialogue with the FDA about more

widespread adoption of green chemistry practices, and been instrumental in planning publication of this dialogue for a broader audience.

Scott Obach, Pfizer, Inc.

We would like to recognize Scott Obach for his sustained leadership in the DMLG. Scott was responsible for taking the old PhRMA drug metabolism discussion group and getting it positioned in IQ. He has been responsible for continuing to foster the interactions with the FDA and cementing the interaction between the DMLG and NCATS.

Qichao Zhu, Boehringer Ingelheim

PSLG Working Group 1 needed to develop a database application to share preclinical safety study data among the member companies. Qichao spent considerable efforts in designing and implementing the database, its application tools, and software testing.

Partha Mudipalli, Teva Pharmaceuticals;

Frank Diana, Endo Pharmaceuticals;

John Brackett

The IQ Contract Manufacturing Working Group was conceived following a 2011 meeting between the IQ Consortium and the FDA at which the FDA asked about the state of contract manufacturing within the pharmaceutical industry. This award is intended to acknowledge the two-and-a-half years of leadership dedicated to this initiative by these three individuals.

2013 SYMPOSIUM ORGANIZING COMMITTEE

Chair, Pierre Boulas, Biogen Idec
Co-Chair, Ben Neuteboom, EMD Serono

Akintunde Bello, Pfizer
Robert Castellucci, Vertex
Donna Clemons, AbbVie
Inge Ivens, Bayer
Thomas Jones, Lilly
Margaret Landi, GSK
Robert McKean, Teva
Ingrid Mergelsberg, Merck
Dennis O'Connor, BI
Lisa Reed, Eisai
Mary Ringwood, BMS
Sarah Robertson, Vertex
Anita Shah, Bayer
Debra Webb, Pfizer
Secretariat, DrinkerBiddle

Lew Kinter, IQ
Consortium Chair
(left) and
Pierre Boulas,
Symposium
Organizing
Committee Chair
(Right)



A GREENER SYMPOSIUM

The overarching theme of this year's Symposium, *Landscape 2020*, invited us to think about the pharmaceutical industry's future and the positive impact IQ can have on science and regulation.

In planning this event, we also thought about the impact IQ has on other elements of our physical and metaphorical landscape, including our environment.

We took several steps to minimize the environmental impact of this event. Among them:

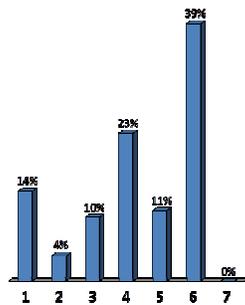
- ◆ Reduced waste by using web-based registration, advertisement, and distribution of materials and choosing china over disposable plates and cups.
- ◆ Reduced transit by selecting a central location accessible by public transit.
- ◆ Serving local, organic foods when possible and donating leftovers.

{Polling Results}

This year's symposium incorporated Audience Response Polling technology to the presentations to allow for live-polling of the audience. For full results, visit iqconsortium.org/news/landscape2020.

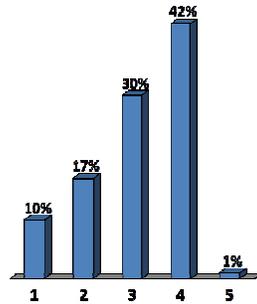
6. From your perspective, the biggest obstacle to Open Innovation in the pharmaceutical industry is:

1. A risk-averse corporate culture
2. People's behavior
3. The organizational structure typically found in companies
4. Intellectual Property protection and licensing agreements
5. A lack of clear vision describing what can be achieved
6. All of the above
7. None of the above



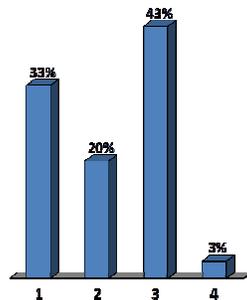
2. For your company's corporate strategy and from your point of view, the development of personalized medicines is:

1. An aspiration (would be nice to have but it is not a priority, i.e. not a lot of time and resources are spent incorporating personalized medicines in development programs)
2. A long term goal (the strategy and roadmap to enable the development of personalized medicines is still in the design stage)
3. A must have (i.e. your company is actively working on making personalized medicine a reality)
4. A reality (i.e. your company has already successfully incorporated the concept of personalized medicine in development programs)
5. I do not know



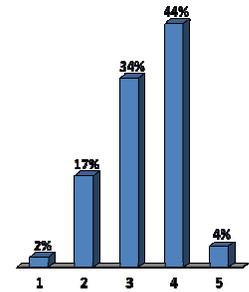
2. From your perspective, Globalization today is:

1. A reality and my company has adapted efficiently to this reality.
2. Disruptive in a negative way because of the burden it creates.
3. Disruptive in a positive way and I welcome the opportunities it provides.
4. Not disruptive as it is not really impacting my daily work.



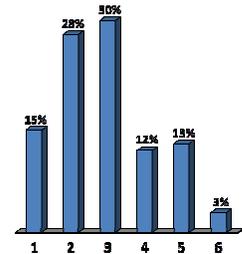
3. Select the statement below that best describes, in your opinion, the collaboration between your company and outside organizations (companies, universities, venture capitalists ...) used currently to advance Innovation:

1. Projects that rely on such collaborations are rarely pursued.
2. These collaborations happen ad-hoc and only when required by specific projects' needs, i.e. their scopes are very limited.
3. Strategic collaborations are encouraged but are difficult to implement because of the cultural, political, and organizational challenges.
4. Establishing such collaborations is an integral part of my company's strategy and is well supported by our business processes.
5. I am not aware of my company's strategy when it comes to outside collaboration.



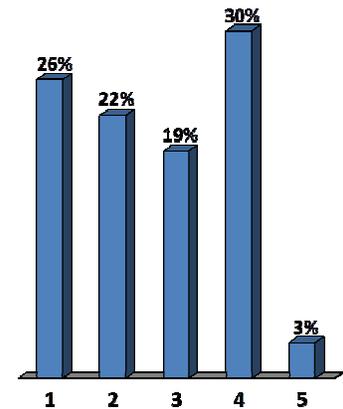
5. In your opinion, the biggest obstacle to the development of personalized medicines is:

1. The cost
2. The identification of patients prospectively and in a timely fashion for trial enrollment
3. The quality of the science or lack of available technology
4. The requirement for parallel development of a companion diagnostic
5. The existing business processes and organization structure
6. Not listed above



6. In your opinion, select the most plausible scenario for our industry in 2020 when it comes to globalization and Cloud Computing:

1. This is going to be exciting: the real-time seamless access of information from anywhere at any time will enable users to engage with each other, operate and innovate in smarter ways.
2. This is going to be challenging: we will be inundated with data and will need new tools to visualize it and extract useful information.
3. This is going to be frustrating: some areas will adopt the cloud (e.g. clinical trial managements, sales and marketing ...) while others will remain grounded on earth (e.g. drug discovery, manufacturing ...) further expanding the gap between the different functions.
4. This is going to be interesting: since adoption of cloud computing is unlikely to be uniform across the industry, our core business processes will be challenged especially when it comes to collaborations with external partners and CMOs.
5. Nothing will really change: The desire to keep everything internal and hold onto it because we want to protect it will prevail. It will be business as usual as in 2013.



The International Consortium for Innovation and Quality (IQ), formed in 2010, is a not-for-profit organization of pharmaceutical and biotechnology companies with a mission of advancing for pharmaceutical and biotechnology products worldwide.