2016 IQ Consortium Symposium Report

The sixth annual IQ Consortium Symposium, “Mission Possible: New Horizons in Drug Development,” was held in Leesburg, Virginia, on October 5th, 2016. Symposium participants spent the day contemplating moonshots that could change the future of drug development, and reflected on ongoing and emerging initiatives that are making previous moonshots reality.

A Fusillade of Moonshots

**Bink Garrison, Bink Inc.**

“Put a man on the moon, and return him safely to earth by the end of the decade.” So commanded President John F. Kennedy in his visionary 1961 address to Congress. Eight years later, the Eagle had landed, and the term moonshot began to find its way into the innovation lexicon.

To kick off the Symposium, IQ invited Bink Garrison, who gave a presentation on innovation at the first IQ Symposium in 2011, to run an interactive session challenging participants to develop moonshots for drug development and innovation. Audience members broke out into respective Leadership Groups (LGs) or those LGs with which they most closely identified. They were challenged to brainstorm on moonshot ideas for their specific group’s disciplines which would be shared with and on which the full audience would vote. The winning ideas covered everything from achieving on-demand, cost-effective, environmentally conscious and timely API delivery to building a virtual human.

**Winning Leadership Group (LG) Moonshot Ideas**

**3Rs**: Eliminate the need of animal testing through alternative technologies

**Analytical**: Unify regulatory expectation to a global perspectives for new molecular entities

**API**: On-demand, cost-effective, environmentally conscious and timely API delivery

**Biologics CMC**: Build an integrated mobile capability that enables delivery of personal medicine

**Clinical Pharmacology**: Radical transformation of clinical trial paradigms

**Drug Metabolism**: Build the virtual human (eliminate animal and human studies)

**Drug Product**: Diagnostic-based, individualized, on-demand drug supply and delivery

**DruSafe**: Map all toxicology species and human biology

**GLP/GMP QA**: Global quality system based on risk-based technology support
Allotrope set out to deliver this framework in three years. To achieve this, Allotrope hired professionals dedicated to the vision of Allotrope. Through the Allotrope Partner Network, Allotrope built relationships with the vendor community and sought feedback on vendor requirements. Today, in addition to the 12 member companies of the Allotrope Foundation, the Allotrope Partner Network Community has grown to over 30 vendor companies, including innovative start-ups.

In 2015, Allotrope reached a major milestone with its first internal release of the Allotrope Framework. The Allotrope Framework that will support the standardization of data representation and enable the creation, use and exchange of laboratory data across proprietary equipment. Forming the foundation of the framework is the Allotrope Data Format (ADF), a new data format that is capable of storing datasets of unlimited size data from any kind of analytical technique in a single file and that uses a semantic platform to store metadata, enabling powerful big data analytics.

Currently, vendors and member companies are collaborating on Allotrope Framework integration projects, developing novel tools for use cases that span the life cycle of analytical data, from managing data across proprietary equipment. Allotrope is preparing for its first public framework release in 2017.

To learn more about Allotrope Foundation, visit http://www.allotrope.org/.

Enabling Technologies Consortium

In the second presentation, Chris Welch from Merck & Co. and Gina Black from Bristol Myers Squibb described the newly formed Enabling Technologies Consortium™ (ETC) and presented the progress of one of the pilot projects.

Enabling technologies are tools that make the work of the pharmaceutical industry easier, but do not necessarily represent the industry's core business. They are found in all phases of the product life cycle from discovery through to development to manufacturing and can be anything from pipettors to gene assays to robotics. When the need for new enabling technology is recognized, a company wait to see if someone else develops it, undertakes development themselves, partner with a vendor to develop it, or form an industry collaboration. The last model can be advantageous because it allows for sharing of cost and risk, but can also present logistical obstacles, such as the long delays associated with the establishing multiple parallel legal agreements. ETC grew out of an IQ Working Group – was established to address these issues and provide a streamlined path to industry collaboration for the development of enabling technologies.

ETC is open to pharmaceutical and biotechnology companies developing new molecular entities for treatment, prevention or diagnosis of disease, and within ETC, a collaborative group can also contract work with commercial vendors or academic organizations under a single legal agreement. The initial focus of the ETC is Chemistry, Manufacturing and Controls (CMC). There are currently seven working groups covering different areas within CMC: Personal Parallel Reactors, Crystallization, Analytical and Purification, HTE Solid Dosing, Drying, Dosing, Mass, Solving.

The progression of one of ETC’s first projects – development of a next-generation preparative Supercritical Fluid Chromatography (SFC) instrument – was described to illustrate how an ETC project progresses. Over the course of four months, in the discussion phase, the project team identified the technology gap and outlined the project scope, must-have features, and project details. In the proposal phase, an RFI was drafted and released based on the consensus requirements, and vendor responses were received. The RFI responses are currently being reviewed, and when a vendor is selected, the project will move to an execution phase. The prep SFC project was selected as a pilot project in part because the project was a tractable and straightforward development idea, which would provide the opportunity to refine ETC’s approach. Notably, experience with the prep SFC project to date has demonstrated that vendors are interested in working with ETC.

To learn more about ETC, visit http://www.etconsortium.org/.

Traveling the Road to Precision Medicine

Jack Whelan

Jack Whelan is a Research Advocate, Patient Advocate and Legislative Advocate surviving and managing a "not yet curable" blood cancer, Waldenstrom’s Macroglobulinemia. Formerly a Wall Street Research Analyst in Information Technology (IT) and career Sales VP in IT, Jack has refocused his work on bridging the communications gap between life sciences, medical professionals and patients, and spoke to IQ about his experience with cancer and perspectives on patient engagement, the concept of patient centrality versus research centrality, and how this is related
to the drug development and clinical evaluation process.

At the outset of his talk, Jack presented the concept of an “e-Patient,” which he sought to become following his diagnosis with cancer. “e-Patients” – initially written about by Tom Ferguson, M.D., in 1975 – are patients who are electronically connected, equipped with skills to help manage their condition, enabled to make self-care choices, empowered to ask relevant questions, engaged in care, equals with involved physicians, expert on their disease, experienced, and educated and credible. Jack emphasized that education was central – and the most important aspect – of this concept, and talked about how he has become engaged and educated through patient advocacy, professional advocacy, and legislative advocacy, which has included participation in the American Association for Cancer Research, among other organizations.

Patient centricity as opposed to research centricity was a primary focus of Jack’s talk. He spoke about the current clinical trials process as being out of balance, with a heavier emphasis on protocols, labs, and research than on patients, clinics, and treatments, and described a more patient centric process – in which these elements would be equally balanced, data would be fully disclosed, and current information technology would be utilized – as something to strive for. Jack encouraged the industry to design protocols that more engage patients, who often both want to help with research and are keenly interested in disease. He also talked about strides being made in precision medicine.

Jack concluded the talk by encouraging those in the life sciences to be less risk averse, and to really think of patients as patients and people – not subjects, members of a cohort, or cases, among other terms – and emphasized his point by sharing photos of him and his family.

**Particle Delivery Technology**

**Neal Fowler, CEO, Liquidia Technologies**

Neal Fowler, the CEO of Liquidia Technologies, described Liquidia’s work to use their novel, proprietary PRINT® Platform to engineer improved therapeutics for patients. By using a templated fabrication, the PRINT Platform enables the creation of a uniform population of particles of defined size, shape and formulation. The technology can be used to make particles ranging from sub-micrometer to millimeter scale in a vast number of shapes. The PRINT approach enables particles to be engineered with very specific features in order to overcome a wide range of specific drug delivery challenges and can be applied to therapeutics for a variety of diseases and different routes of delivery (e.g., inhaled, oral, topical). Mr. Fowler described the application of the technology in three disease areas – pulmonary arterial hypertension, pain management, and glaucoma – to develop therapeutics with improved delivery characteristics more acceptable to patients. By providing precise control over the design of the particles and enabling the production of particles with characteristics not previously possible, Liquidia’s PRINT platform technology provides new opportunities in therapeutics development.

**Drug Development for Global Health – Challenges and Opportunities**

**Niya Bower, The Bill and Melinda Gates Foundation**

The Bill and Melinda Gates Foundation is a well-known foundation with global reach. With a more than $40B trust endowment, 1,500+ active grantees, and 1,400+ employees worldwide (2015), the Foundation is taking an integrated approach to improving people’s lives, with programs in global health, global development, policy and advocacy, communications, and more.

For the IQ Symposium, Niya Bower, Senior Program Officer, CMC, Integrated Development, centered her talk on the Gates Foundation’s global health activities. She described the mismatch that exists between the burden of disease globally, availability of medical care, and biomedical research, as well as the lack of new infectious disease drugs which makes closing gaps in global health so difficult. To help address these challenges, the Gates Foundation has invested in a diverse therapeutics portfolio. Among other achievements, they have successfully piloted a low-cost API manufacturing process, and developed the Sayana Press, which provides contraceptive protection in an easy-to-administer format.

**Visualizing the Future: Part II**

**Microphysiological Systems**

Brian Berridge, of GlaxoSmithKline, the Vice Chair of the 3Rs Leadership Group and Microphysiological Systems (MPS) Working Group leader, presented an introduction to the MPS to open a panel discussion on the future of these systems. Joining Dr. Berridge on the discussion panel were Szczepan Baran, Peggy Guzzie-Peck, of Johnson & Johnson, and Jeetu Eswaraka of Amgen.

MPS, or tissue chip systems, are engineered platforms that aim to replicate the biology in animal systems and to provide a complex, human-relevant in vitro model system. Dr. Berridge provided an overview of the MPS WG’s collaboration with the National Center for Advancing Translational Sciences (NCATS) to develop a strategy to get MPS into industrial settings in meaningful and impactful ways. NCATS and Defense Advanced Research Projects Agency (DARPA) have both invested heavily in MPS technology, and this investment has been accompanied by a rapid proliferation of commercial MPS technologies. The technology is at a critical phase in its development, and there is an opportunity for the pharmaceutical industry to drive the maturation of the technology.

Ultimately, the hope is that MPS will be able to outperform current animal models in preclinical development. However, along with the great promise associated with incorporating MPS systems into drug development comes a myriad of questions and challenges, including defining standards for measuring and evaluating the performance of these systems and accounting for the biology that is not captured in the MPS.

The panel discussion focused on questions related to defining the context of use for these systems within industry; understanding how to interpret the mechanistic endpoints provided by the MPS models and their relationships to current toxicology models; and building confidence in the results that are...
batch manufacturing; for instance, how does one or how to handle internal QA in CM. Another such start-up and shut-down processes of the instrument.) In some cases, switching to CM can require new slowed its adoption by the pharmaceutical industry. Despite the benefits of continuous manufacturing also offers supply chain flexibility that in turn can also support more versatile, adaptive manufacturing ? Nevertheless, experience with continuous manufacturing at Lilly suggests that the benefits far outweigh the challenges. Continuous manufacturing may also be an area of opportunity for IQ, an area that could benefit from efforts to develop consistent best practices or recommendation to address these concerns and support the adoption of continuous manufacturing.

I.Q. Recognition Awards
3Rs Leadership Group (3Rs LG)
Letty Medina, AbbVie, Chair, CRO Outreach Working Group
Dale Martin, Sanofi, Leadership, Management and Design of Global 3Rs Award in collaboration with ALAAAC International
Analytical Leadership Group (ALG)
Nara Variankaval, Merck & Co., Chair, Co-crystal Working Group
Clinical Pharmacology Leadership Group (CPLG)
Jinshan Shen, Vertex Pharmaceuticals, Chair, Clinical Pharmacology Guidance Committee
Paulien Ravenstijn, Janssen, Chair Pediatrics Working Group
Drug Metabolism Leadership Group (DMLG)
Fabio Broccatelli, Genentech, Co-chair, In-silico ADME Discussion Group
Prashant Desai, Eli Lilly and Company, Co-chair, In-silico ADME Discussion Group
Drug Product Leadership Group (DPLG)
Tzuchi Rob Ju, AbbVie, Chair, Drug Product Leadership Group and Co-chair, Pediatric Formulations Working Group
Robert Ternik, Eli Lilly & Company, Co-chair, Drug Product Leadership Group and Pediatric Formulations Working Group
Good Manufacturing Practice Quality Assurance Leadership Group (GLP/GMP QA)
Dennis O’Connor, Boehringer Ingelheim, Chair, GMP QA Leadership Group, Change Management in Development Working Group, 2016 and Co-chair 2015 IQ Symposium Organizing Committee
DruSafe
Sherry Ralston, AbbVie, Co-lead, Attrition Data from Pre-FIH Animal Toxicity Studies Focus Group
Ken Loveday, Biogen, Co-lead, Attrition Data from Pre-FIH Animal Toxicity Studies Focus Group
Dolo Diaz, Genentech, Co-lead, Attrition Data from Pre-FIH Animal Toxicity Studies Focus Group
Mike Graziano, Bristol-Myers Squibb, Lead, Mini-pigs in Safety Testing Focus Group
Tony Arulanandam, Alexion, Co-lead, FDA BioSafe Annual Meetings
Lorrence Buckley, Eli Lilly and Company, Co-lead, FDA BioSafe Annual Meetings
Debi Hoivik, Boehringer Ingelheim, Co-lead, FDA BioSafe Annual Meetings
Tom Jones, Eli Lilly and Company, Co-lead, FDA BioSafe Annual Meetings
Diann Blanset, Boehringer Ingelheim, Co-lead, FDA BioSafe Annual Meetings
Max Derzi, Pfizer, Lead, Cross-functional (DruSafe/ DMLG) Commenting Groups 1) EMA concept paper for FIH clinical trials and 2) recent FDA regulations on GLP Modernization

Our Mission: The IQ Consortium is dedicated to augmenting the capability of member companies to develop transformational solutions that benefit patients, regulators and the broader R&D community.
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