Evolving Enabling Technologies Across CMC

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2014 IQ Symposium: Innovation Through Precompetitive Collaboration

IQ Enabling Technologies Working Group

Motivation:

- Optimization of **Technologies that Enable** pharmaceutical development will increase efficiency and effectiveness of key CMC activities.
- The optimization can be achieved by **pre-competitive collaborations** among IQ member companies.

End Goal:

- To identify existing technology gaps in the CMC space and enable cost-effective and relevant solutions to improve efficiency and quality in the research and development process.

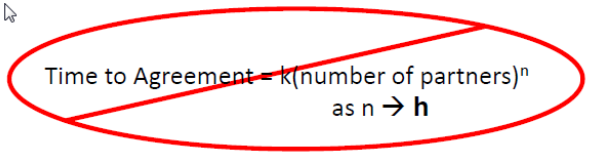
Assessment of Precompetitive Collaborations

Advantages

- Introduces Efficiency: ability to influence potential solution providers to address unmet/future needs
- Minimizes financial impact
- Opportunity to share best practices
- Leverages broad SME pool

Challenges

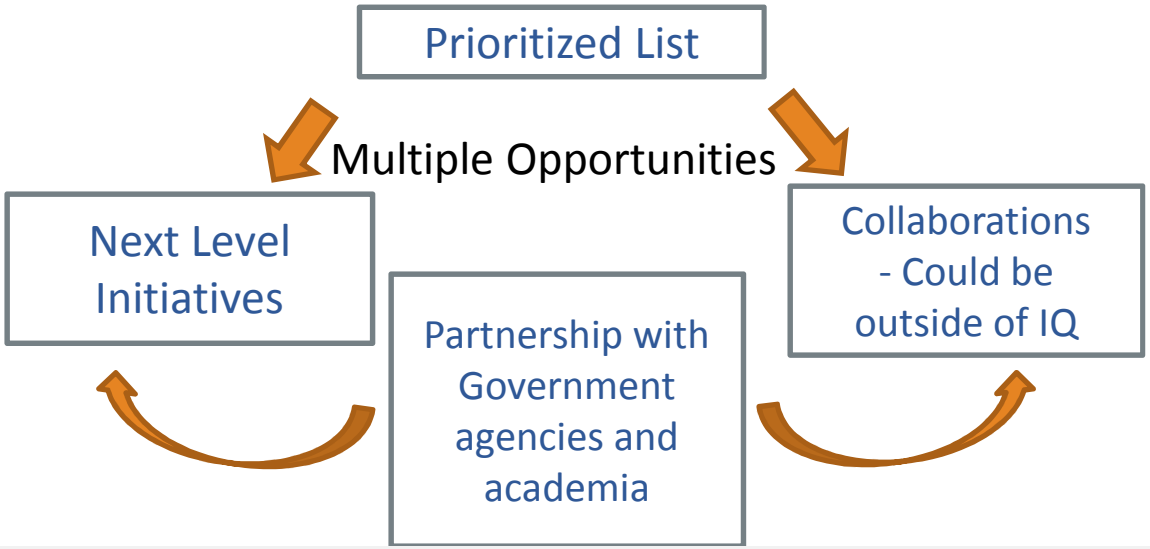
- Logistical and Managerial complexity in management of consortia



- Ability to deliver results quickly
- Managing- IP-to maintain incentives for commercialization or publication
- Average (or sum) of group's desires may not fit anyone's requirements
- Balanced and transparent collaboration

Phase 1: Establishing what is pre-competitive

- What is in pre-competitive space will help define how collaboration/participation will work.
- Need list of opportunities and areas for collaboration.



- Identified 5 areas of initial focus (Automation, PAT, Modeling, Crystallization & Flow Chemistry) and defined scope for Wave 1.
- Established prioritized list through identification and active interaction with pre-competitive collaboration 'champions' from **13** member companies.

Defining the Opportunity and Scope: Preliminary Analysis (Modeling)

		Company												
Area	Score (unweighted)	1	2	3	4	5	6	7	8	9	10	11	12	13
DOE/DOE linkages -kinetics	39	0	6	6	8	0	1	0	7	5	0	0	0	6
Retrosynthesis	12	0	6	0	0	0	0	0	0	0	0	6	0	0
Computational Chemistry Algorithms	7	1	0	0	0	0	6	0	0	0	0	0	0	0
LogD, pKa, solubility	29	2	0	0	0	0	10	0	7	0	2	0	0	8
Modeling of distillation, filtration, reaction kinetics	35	0	0	6	0	0	2	3	6	0	6	6	0	6
Adsorption, chromatography	8	0	0	0	0	0	8	0	0	0	0	0	0	0
Predictive Tools for Chemical Properties	33	7	7	9	6	0	4	0	0	0	0	0	0	0
In-silico tools for Process Modeling (CFD, mixing, kinetics)	50	10	7	8	2	0	1	0	10	0	2	0	0	10
Crystallization	52	3	8	0	7	0	9	0	10	0	7	8	0	0
Particle shape modifiction/prediction, g	38	0	8	0	0	0	10	6	10	0	0	0	0	4
Drying	52	8	0	0	8	10	7	0	10	0	0	0	0	9

Phase II. Defining the Operating Model

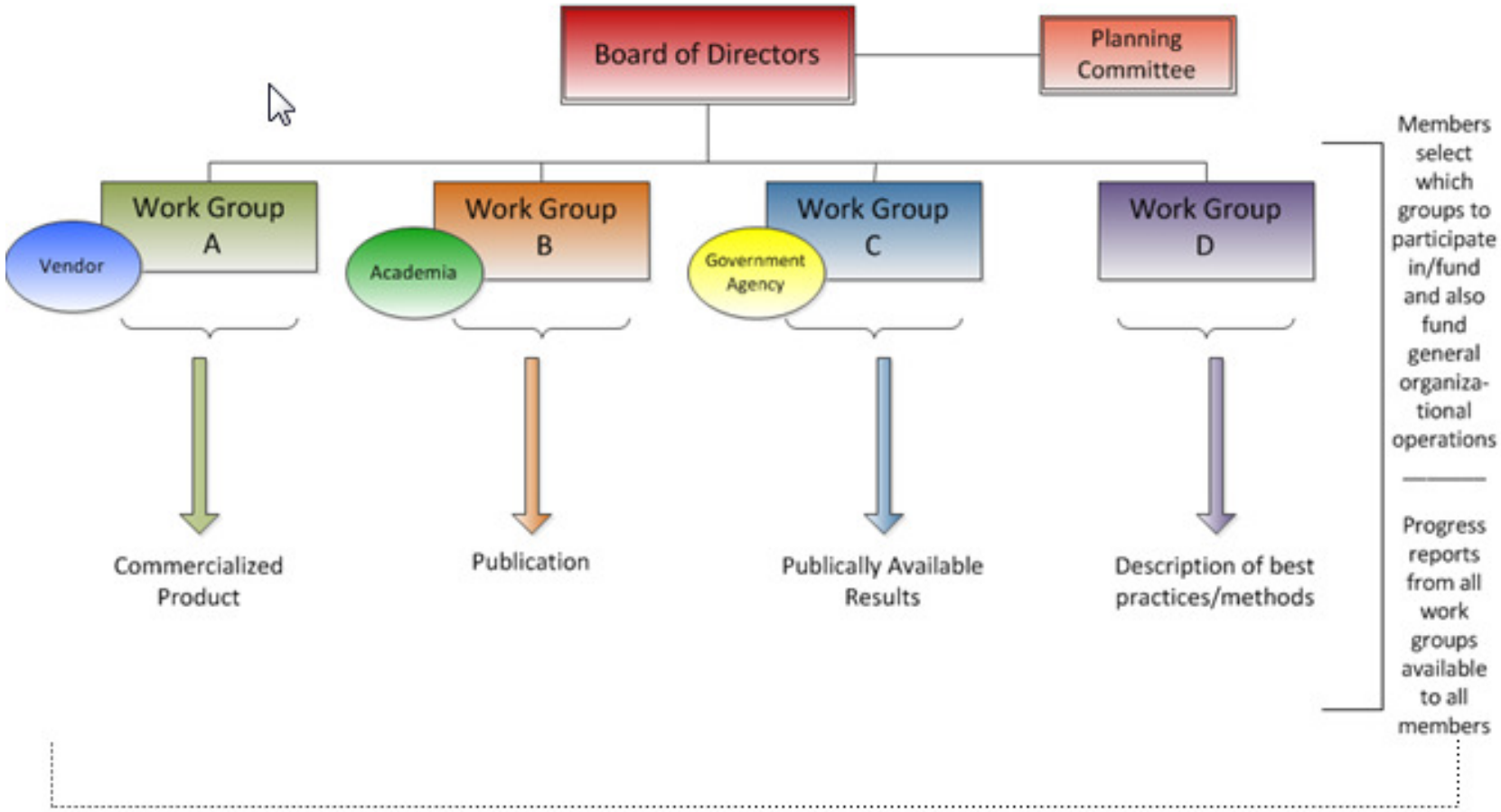
IQ's role as the 'honest broker' to facilitate efficient collaborations.

- Foster sharing of ideas related to enabling laboratory technologies while maintaining protection of IP rights, so as to maintain incentives for commercialization and publication.
- Enable opportunities ranging from: sharing of knowledge/best practices, carrying out detailed assessment/provide targeted feedback to academic or vendor collaborations.

Aim to gain small wins and deliver results quickly

- Identified 8 project proposals with limited scope for Phase 2.
- Several projects put forth and ***accepted as topics*** for SBIR grant proposals.
- Projects to be staged/have multiple phases – ranged from simple/easy to complex implementation.

Phase III: Enabling Technologies Consortium
Proposal



Alignment and Engagement

Alignment

- Identified closely aligning efforts
  - Leveraged current state of thinking on pre-competitive models* as a starting point.
  - Adopted best practices/key learnings from other efforts in this space
  - Update provided to CCR Annual Meeting

Engagement with Government/Academia

- NIH – NCATS , Discussion at recent NSF sponsored workshop on data-driven chemistry

* **Precompetitive Collaboration on Enabling Technologies for the Pharmaceutical Industry**, Welch, Christopher J.; Hawkins, Joel M.; Tom, Jean Org. Proc. Res & Dev (2014), 18(4), 481-487.

IQ Enabling Technologies: Working Group Members

- Srinivas Tummala (BMS)
- Jose Tabora (BMS)
- David Askin (Genentech)
- Margaret Faul (Amgen)
- Gang Xue (Amgen)
- Partha Mudipalli (Teva)
- John Traverse (Celgene)
- William Kiesman (Biogen)
- Joel Hawkins (Pfizer)
- Shailendra Bordawekar (Abbvie)
- Steve Wittenberger (Abbvie)
- Christopher Welch (Merck)
- Aaron Cote (Merck)
- Charles Papageorgiou (Takeda)
- Kevin Siebert (Lilly)
- Chris Senanayake & Jeff Song (BI)
- Frank Roschangar (BI)
- Darryl Ertl (GSK)
- Christian Airaiu (GSK)

Backup Slides

Identification of the Operating Model

Defining the Model(s) that best reflect(s) the opportunities

- Pharma companies share existing technology
  - Members brainstorm new technology to be commercialized by a vendor
  - Partnering with academic researchers to develop and explore new models
- Models can be grouped around specific technologies where ideas can be discussed and exchanged subject to ground rules about IP
  - Vendor led, Multi-company collaboration, Joint Venture to manage, “Honest Broker” (IQ, CCR)

Develop Technology via *Balanced* Collaborations

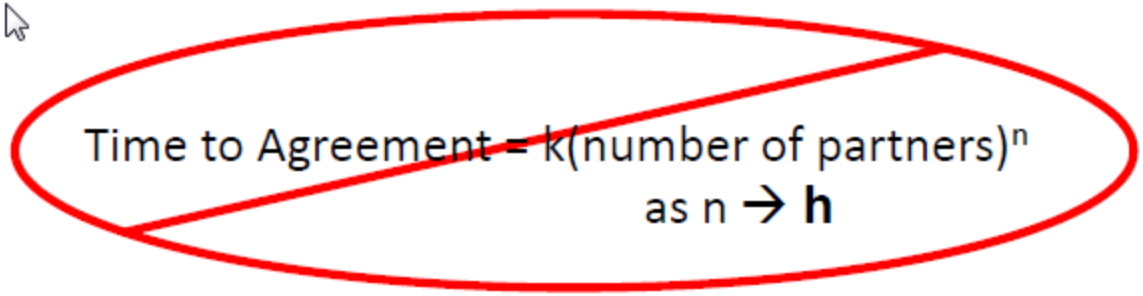
- Develop and Deliver technology via *openly established and transparent* collaborations
  - Share cost between pharma (& vendors where appropriate).
  - Share risk, i.e. to develop a proof of concept.
  - Size it right, enough collaborators to bring value but not too many to manage.
  - Have a clear understanding of everyone's goals.
  - Choose partners through open and objective process.
  - Develop trust through the creation of appropriate confidentiality arrangements.

Delivery and Communication of Results

- Gain small wins for *Big Successes*
  - Start with smaller opportunities to benchmark success.
  - Work in parallel with independent spends/agreements but share information as appropriate.
  - Agreement around single project with shared funding.
  - Agreement around multiple projects with shared funding.
  - Shared entity with laboratory attached.
  - Need good oversight to ensure efficient operation.

Timeframe to Establish an Operating Model

- Ability to deliver results quickly



- Enable more efficient collaboration thru a standardized legal framework that allows projects to advance quickly.
- Agree on a timeline for completion of the legal Agreements at onset.
- Assign accountable points of contact to progress this timeline.
- Understand everyone's goals and expectations from the start.

Complying with Antitrust Requirements

- Understanding that participants in a precompetitive collaboration may compete at many levels.
- Create a framework to monitor collaborative activities to comply with antitrust laws.
- Must educate participants in a collaboration on antitrust requirements.

Defining the Operating Model

- *(Challenge 2 and 3) Defining operating models to*
  - enable appropriate conversations which may have confidentiality/IP considerations, sharing of knowledge/best practices, carrying out detailed assessment/provide targeted feedback, academic or vendor collaborations
  - IQ role as the 'honest broker' – facilitate efficient collaborations
- *(Challenges 4 and 5) Aim to gain small wins and deliver results quickly*
  - Identified 8 project proposals with limited scope for Phase 1
  - Couple of projects put forth and accepted as topics for SBIR grant proposals - **Simple and Robust Reaction Progress Analyzer & Online Real Time Metals Analysis at Low ppm Level**
  - Projects to be staged/have multiple phases – ranging from simple/easy to complex to implement.

Slide 16

2 Not clear what these challenge numbers reference, where is 1 and 2?
Robyn Rourick, 9/30/2014

3 How is this facilitated?
Robyn Rourick, 9/30/2014

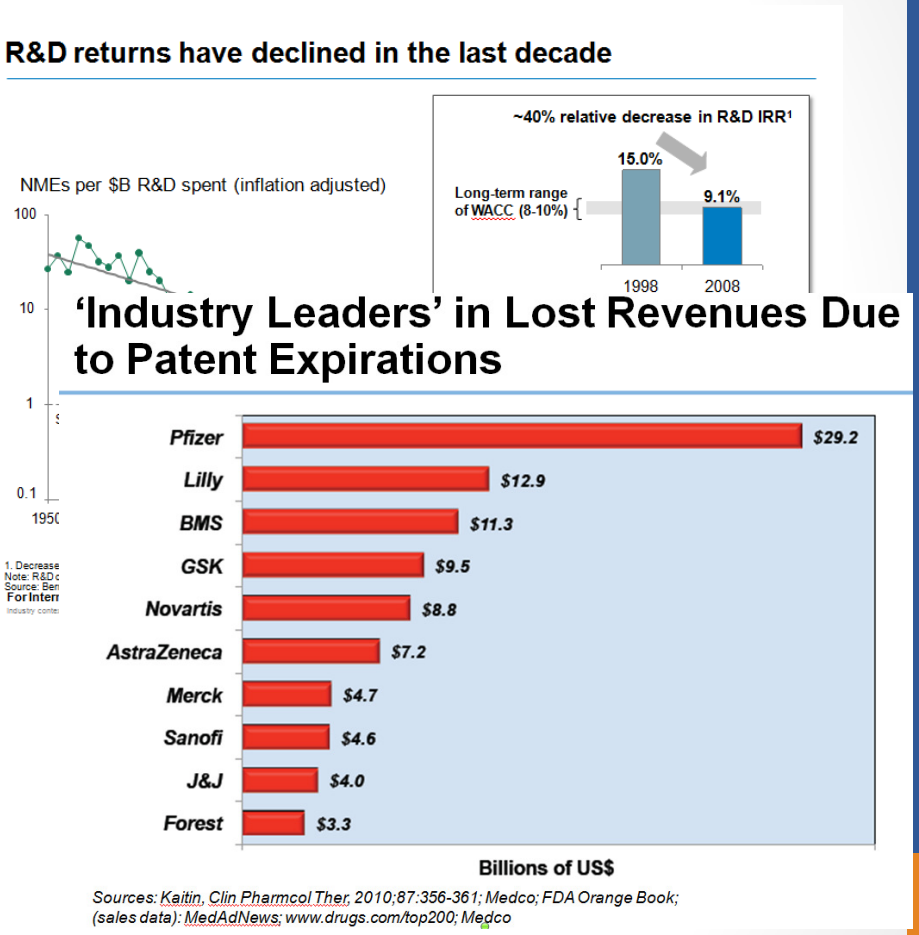
What is a “Precompetitive Collaboration?”

For the purpose of this presentation, a “precompetitive collaboration” will mean:

- a collaboration between two or more pharmaceutical companies, potentially including academics, government agencies, or vendors.
- which is designed to produce an efficiency-enhancing advancement or refinement that will be made broadly available to the public, either through publication, commercialization of a new product, or other means.
- in which the pharmaceutical companies will retain no proprietary interest.

Strategic Imperative for Precompetitive Collaborations

- Internal R&D under pressure to deliver new therapeutics more efficiently.
- Patents on many top-selling products are expiring.
- Cost to meet safety and efficacy is rising due to increased regulatory hurdles.
- Growing need to get new drugs to treat rare diseases and diseases in developing countries.
- Pharmaceutical R&D remains a long, risky, and expensive process.



What opportunities lie in the Precompetitive Space?