Pre-Competitive Collaboration in Clinical Trials

July 24, 2013
Garry A. Neil, MD
Board of Directors, Chairman
R&D leaders identified Clinical Trial Execution as a key priority area for industry-wide collaboration

**Survey Results**

On Value…

“Sharing of infrastructure is an opportunity.”

“Clinical trials is by far most important. Challenges include regulation, globalization, complexity . . .”

“We have already implemented major changes in clinical operations, but we are interested in more "industrial" trial site execution.”

On Doability…

“Requires regulator endorsement.”

“Sharing of infrastructure is a major opportunity.”
Mission Statement

“TransCelerate BioPharma will develop shared industry research and development solutions to simplify and accelerate the delivery of innovative products to patients. Our non-profit, pro-competitive model will be based on a results-oriented approach, emphasizing increased quality in clinical trials and improved patient safety, enabled by broad participation and collaboration across the global research and development community.”
A relatively flat organization structure has been developed to manage projects and operational activities.

TransCelerate BioPharma, Inc.

- Board of Directors
  - External counsel
  - Accounting Firm
  - Audit Firm
  - Administrative Asst.

- CEO
  - Operations Committee
    - Program Management Office
      - Director of Operations
      - Director of Projects
      - Architecture and Design
      - Project Value / ROI
      - Change Management

Operations Subcommittees:
- Communications
- Regulatory Council
- External Engagement
- New Member Engagement
- Future Initiatives Planning

Project Workstreams:
- Architecture and Design
- Project Value / ROI
- Change Mgmt

Key:
- Retained position
- Contracted resources
- Member representatives

Architecture and Design, Project Value / ROI, and Change Management will work across all five TransCelerate projects.
The Charter Members of TransCelerate include Major Biopharmaceutical Companies

**John Leonard** (Board Member)
*VP, Chief Scientific Officer*

**David Jordan** (Operations Committee)
*Divisional VP, Stats & Data Mgmt*

**Briggs Morrison** (Board Member)
*EVP, Global Medicines Development*

**Sue McHale** (Operations Committee)
*Executive Director, Global Project Delivery*

**Klaus Dugi** (Board Member)
*Corporate SVP, Medicines*

**Thor Voigt** (Operations Committee)
*Head of Global Clin Ops, Biometrics & Data Mgmt*

**Brian Daniels** (Board Member)
*VP, Global Development & Medical Affairs*

**Jonathan Zung** (Operations Committee)
*Vice President, Global Development Operations*

**Patrick Vallance** (Board Member)
*President, Pharmaceuticals R&D*

**Lynn Marks** (Head of Operations Committee & Secretary)
*SVP, Clinical Platforms & Sciences*

**Pete Milligan** (Operations Committee)
*Director, Lead for SCD*

**Paul Stoffels** (Board Member)
*Worldwide Chairman of the Pharma Group*

**Martin Fitchet** (Ops Committee & Treasurer)
*SVP Projects, Clinical Platforms & Sciences*

**Jan Lundberg** (Board Member)
*EVP of Science and Technology*

**Jeff Kasher** (Operations Committee)
*VP and COO Global Medical R&D*

**John Hubbard** (Board Member)
*SVP Development Operations*

**Craig Lipset** (Operations Committee)
*Head of Clinical Innovation*

**Corsee Sanders** (Board Member)
*Global Head of Development Innov. & Clin Ops*

**Carol Harris** (Operations Committee)
*Global Head Project & Functional Excellence*

**Elias Zerhouni** (Board Member)
*President of Global R&D*

**Ji Zhang** (Operations Committee)
*Head of R&D Scientific Platforms*

**Andy Lee** (Operations Committee)
*SVP, Head Global Clin Ops, Genzyme*
Seven new member companies have joined TransCelerate in the first half of 2013

Sef Kurstjens (Board Member)
CMO & President Pharma Global Development

Nancy Sacco (Operations Committee)
Executive Director, Development Sciences/Strategic

Garry Neil (Chairman of the Board)
Head of R&D

Marco Taglietti (Board Member)
President, Forest Research Institute & CMO

Ulo Palm (Operations Committee)
SVP, Clinical Operations & Biometrics

Steve Gilman
EVP, R&D and CSO

Ed Campanaro (Operations Committee)
VP, Clinical Development

Alfred Sandrock (Board Member)
SVP, Head of Development Sciences & CMO

Murray Abramson (Operations Committee)
VP, Global Clinical Operations

Annalisa Jenkins (Board Member)
Head of Global Development & Medical

Kathleen Ford (Operations Committee)
Senior VP, Head of Global Clinical Operations

Pablo Cagnoni (Board Member)
EVP, Global Head of R&D

Barbara Klencke (Operations Committee)
SVP, Clinical Development
Five Clinical Trials Initiatives

Five opportunities in clinical trial execution were prioritized for action based on industry readiness and ability to execute in 2013.

Initiate Immediately
Deferral Start
(initial ideas subject to change)

Value to R&D

Now (2013)
Later (Q4 2013+)

Highest

Provide comparator drug (marketed products used in clinical trials)
Clinical Data Standards
Standard Approach for High Quality, Risk-Based Monitoring
Investigator / Site Portal
Centralize / Share Site Qualification and Training

Lowest

End-to-End Data Flow
Deliver Label / Drug Information via Mobile Device
Placebo/Standard of Care Data Sharing
Metrics and Performance Data Sharing
Develop Investigator Networks & Minority Population Site Recruitment

Five opportunities in clinical trial execution were prioritized for action based on industry readiness and ability to execute in 2013.
## Five Selected Areas of Focus Have the Shared Goals of Increased Quality, Patient Safety and Accelerated Development Timelines

<table>
<thead>
<tr>
<th>Initiative</th>
<th>Objective</th>
<th>Benefit</th>
<th>Progress to date</th>
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| **Standardized Approach for High-Quality, Risk-Based Monitoring** | Develop a standard framework for targeted, risk based clinical trial monitoring | • Improvement in data quality and patient safety for clinical trials  
• Reduction in effort expended on low-value activities | • FDA and EMA feedback incorporated  
• Position paper and methodology published in May 2013 for access to entire clinical trial community  
• Multiple pilot studies identified for launch in Q3 and Q4  
• Over 1000 unique downloads of position paper |
| **Shared Investigator Site Portal** | Establish a single, intuitive interface for investigators | • Ease of use and harmonized delivery of content and services for investigators  
• Reduce site burden  
• Reduce member company costs | • Defined components to leverage from existing member portals  
• Potential solutions options provided to TransCelerate Board of Directors  
• Board approval to proceed with development in June  
• RFI to identify systems integrator, product partner, and hosting partner underway |
| **Shared Site Qualification and Training** | Mutual recognition of GCP training and site qualification between pharmaceutical companies | • Improved quality of clinical sites and accelerated study start-up times  
• Reduce site burden | • Established standards for mutual recognition of GCP training  
➢ Minimum content elements  
➢ Process for awarding certificates  
• Discussion underway on site qualification document standardization options  
• Mutual recognition agreements approved among members  
• Press release issued in June 2013 |
Five Selected Areas of Focus Have the Shared Goals of Increased Quality, Patient Safety and Accelerated Development Timelines

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| **Clinical Data Standards – Efficacy (in Partnership with CDISC and CFAST)** | Accelerate current efforts underway through CDISC to establish efficacy data standards | Increased quality of clinical data and enablement of industry end-to-end data flow | • Asthma and Diabetes selected as first TAs of focus  
• Asthma scoping draft issued end of Dec 2012; charter approved by steering committee  
• Diabetes project resources identified  
  ➢Scoping ongoing as well as discussions with FDA (applying lessons learned from Asthma)  
• SHARE environment press release issued with CDISC in June 2013 |
| **Comparator Drugs for Clinical Trials** | Establish a supply model to source comparator drugs between companies for use in clinical trials | • Reduce the cost and effort for comparator drug sourcing  
• Reduce the chance of counterfeit drug in study supply chain  
• Share critical data – like solid dose ambient temp excursions | • Determined in-scope products and required documentation for distribution model  
• Defined principles and process for drug distribution model  
• MSA’s between members to be finalized in July  
• First set of transactions expected in Q3 2013 |
External Engagement with the Larger Ecosystem

Outside organizations, including regulatory, public, government and industry-based entities, are being engaged. The intent is not to recreate, but rather partner with existing collaborations.
Key Accomplishments to Date

<table>
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<tr>
<th>Top Accomplishments to date</th>
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<tbody>
<tr>
<td>1 Mobilized 10 companies to create TransCelerate</td>
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<td>2 Created a lean and functional infrastructure of a not for profit entity</td>
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<tr>
<td>3 Added 7 new companies in first half of 2013</td>
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<tr>
<td>4 Initiated two pilots for clinical data standards and published SHARE environment</td>
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<td>5 Published the criteria for mutual recognition of GCP training</td>
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<td>6 Published the framework and approach for risk based monitoring</td>
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<td>7 Launched pilot studies for RBM across multiple members and TAs</td>
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<td>8 Engaged multiple organizations – CCTI, SCRS, BIO, IOM, NIH, ACRO, iMi, etc</td>
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Key Upcoming Milestones

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<td>1 First transaction of comparator drugs among member companies</td>
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<td>2 Initiate standards development of 5 additional therapeutic areas</td>
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<td>3 Expand framework for site qualification recognition beyond GCP training</td>
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<td>4 Launch the first release of the Shared Site Portal</td>
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<td>5 Initiate new projects and expand scope on some existing projects</td>
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Additional Opportunities to Enhance R&D Effectiveness

- Pre-Clinical
  - Target Identification
  - Target Validation

- Clinical
  - Clinical Endpoints
  - Biomarkers
  - Trial Execution

- In Market
  - Post-approval safety & efficacy
Urgent Need for New Clinical Trial Endpoints

Predictive: Survival, Morbidity, relevant to patients (QOL)

Reliable: sensitive and specific, reproducible

Simple: non-invasive, inexpensive, geography independent if event-driven
Need for New Endpoints

Examples

PAH, MD
Sickle Cell Anemia
Alzheimer’s Disease
Stroke
Critical Features of Endpoints

- Clinically Relevant
- Meaningful to Patients
- Meet Regulatory Standards
How do we get there?

- Clinical Trials (Shared)
- Literature
- Thought leader/clinical input
- Patient input
How might we get there?

Consolidated Inputs
- Consensus Conferences
- Whitepapers
- Meta analyses

Testable Hypotheses
- Clinical/Lab/Imaging/Biomarkers
- Composite

Validate Prospectively
- Working Clinical/Regulatory Endpoints
Patient-Focused Drug Development Overview

FDA is developing a more systematic way of gathering patient perspective on their condition and available treatment options

– Patient perspective helps inform our understanding of the context for the assessment of benefit-risk and decision making for new drugs

– This perspective could contribute more broadly to drug development efforts, for example, supporting FDA’s guidance on clinical trial design

• Patient-Focused Drug Development is part of FDA commitments under the fifth reauthorization of the Prescription Drug User Fee Act (PDUFAV)

– FDA will convene at least 20 meetings on specific disease areas over the next five years

– FDA expects that patients, patient advocates, drug developers, and others will attend

Edward Cox, FDA June 13, 2013
Thank You!