TransCelerate's Role in Transforming Pharmaceutical Trials
Presentation to PCORNet

Dalvir Gill, PhD - Chief Executive Officer
17 October, 2014
Presentation Objectives

- TransCelerate History
- Participating Member Companies
- Projects & Accomplishments
- Future Vision
- Q&A - how we can collaborate
Pharmaceutical R&D Leaders Identified Collaboration As A Key Opportunity For Generating Industry-wide Efficiencies

Conducted an industry survey on areas amenable to collaboration
Existing collaboration organizations within the life sciences industry were evaluated for their ability to successfully execute the selected opportunities in clinical trial execution and it was determined that no existing vehicles met the necessary criteria. Therefore Transcelerate was formed to promote a high-level of member accountability and a results-driven approach.

The new organization embodies the following defining characteristics:

- Broad industry membership from Pharma and Biotech
- Lean, non-profit entity with sufficient funding by member companies
- High level of member company control and accountability
- Board of Directors composed of senior R&D leadership
- Member FTE contributions of experienced and skilled resources
**TransCelerate is a Not For Profit Entity Created To Drive Collaboration**

**Vision**
To improve the health of people around the world by accelerating and simplifying the research and development of innovative new therapies.

**Mission**
To collaborate across the global research and development community to identify, prioritize, design and facilitate implementation of solutions designed to drive the efficient, effective and high quality delivery of new medicines.

**Our core values**
+ Quality
+ Transparency & Openness
+ Trust & Integrity
+ Collaboration
+ Courage
19 Pharmaceutical Companies* providing their best talent to collaborate and develop solutions to overcome industry inefficiencies

* 10 Pharmaceutical Companies chartered TransCelerate and 9 additional companies joined in 2013/2014
TransCelerate is often a “catalyst” to open the doors for collaboration amongst industry groups outside of TransCelerate.

The intent is not to recreate, but partner whenever feasible.
Five Opportunities Were Chosen For Action Based On Industry Readiness And Ability To Execute In 2013

1. Model Approach for High-Quality, Risk-Based Monitoring

2. Shared Site Qualification and Training

3. Shared Investigator Platform

4. Clinical Data Standards – Efficacy  (in partnership with CFAST)*

5. Comparator Drugs for Clinical Trials

*Original initiative which began in 2012
Model Approach for High-Quality, Risk-Based Monitoring

Unmet Need: No model framework existed to provide implementation considerations for organizations to successfully adopt and scale the risk-based monitoring methodology

Objective: Develop Guidelines for targeted, risk based clinical trial monitoring

Benefits: Improvement in data quality and patient safety for clinical trials; reduction in effort expended on low-value activities
Shared Site Qualification and Training

**Unmet Need:** Disparate and redundant GCP training, and collection of non-study specific information, are pain points for investigators and sites, as well as for biopharmaceutical companies.

**Objective:** Program established for mutual recognition of GCP training and site qualification credentials.

**Benefits:** Realization of improved quality of clinical sites and accelerated study start-up times.
Unmet Need: Communication between sponsors and clinical trial sites are often cumbersome and difficult requiring investigator sites to use many different websites / login credentials to communicate with their sponsors.

Objective: Establish a single, intuitive interface for investigators to use across the industry.

Benefits: Ease of use and harmonized delivery of content and services for investigators and sponsor companies.
Clinical Data Standards - Efficacy (in partnership with CFAST)

Unmet Need: Clinical data is reported in individual trials in various ways, without benefit of an industry-wide set of standards

Objective: Accelerate current efforts underway through CFAST to establish therapeutic area (efficacy) data standards

Benefits: Increased quality of clinical data and enablement of industry end-to-end data flow
Comparator Drugs for Clinical Trials

Unmet Need: Mechanisms to acquire clinical trial comparator drugs and co-therapy drugs were inefficient and unpredictable; supply chain intermediaries frequently decreased security, introduced delays, and couldn’t provide access to key drug documentation.

Objective: Establish a supply network to source comparator drugs between companies for use in clinical trials.

Benefits: Enhanced patient safety due to known product source and acceleration of study timelines.
Through innovation and collaboration, TransCelerate has accomplished much success in two years

<table>
<thead>
<tr>
<th>Workstream</th>
<th>Accomplishments</th>
</tr>
</thead>
</table>
| **Risk Based Monitoring**         | - The published methodology has received [2,000 + unique downloads](#) from the website  
- The RBM Framework is currently being utilized by multiple member companies with [over 40 ongoing studies launched](#), including 8 ongoing pilots in collaboration with the FDA |
| **Site Qualification & Training** | - Over [62,000 GCP training certificates](#) have been issued by 7 member companies  
- 12 member companies have participated in over 100 GCP training courses submitted by 88 external training providers who are eligible for mutual recognition |
| **Shared Investigator Platform**  | - The Shared Investigator Platform (SIP) workstream finalized scope and requirements for the Platform which will allow investigators and site staff a [single point of access](#) to harmonized processes across multiple clinical trial sponsors. |
| **Clinical Data Standards**       | - The SHARE Environment (metadata repository, which will be a [central place to store, manage, and govern data standards](#)) has been deployed  
- Published data standard user guides for Asthma, Alzheimer’s, Multiple Sclerosis and Diabetes and plans to publish three more by end of year |
| **Comparator Drugs for Clinical Trials** | - The network has successfully [completed over 40 commercial product transactions](#) and completed 60 product document exchanges |
Current State

- Disconnected interfaces
- Manual processes and interventions
- Limited standardization
- Lot of customization
- Rework
- Variable quality
- Wait time
- Missing information
- High costs
- Long cycle times

Future State

- Patient-centric clinical trial design
- End-to-end electronic data flow
- Seamless interfaces
- Automated
- Transparent
- Standardized
- Less rework
- Quality by design
- Shorter cycle times
- Cost efficient
- Integration of Regulatory, Safety, and Medical Sciences
- Conducting clinical trials together
- “Colossal Data Analytics”
Six new project were added in 2014 that complement the Portfolio and support accelerating and simplifying the research and development of innovative new therapies.

#### 6
Common Protocol Template

#### 7
Investigator Registry

#### 8
Pediatric Trial Efficiencies

#### 9
Clinical Trial Diversification

#### 10
Clinical Data Transparency

#### 11
Quality Management System
Common Protocol Template

**Unmet Need:** There is increasing complexity in clinical protocols as well as a lack of consistency within and across sponsors.

**Objective:** Create a common template for clinical protocols to ease interpretation & enable down-stream automation of many clinical processes; develop industry-wide & regulator accepted endpoint definitions.

**Benefits:** Enhance quality of study conduct, facilitate data interpretation and enable down-stream automation of many clinical processes.
Investigator Registry

**Unmet Need:** Sponsors invest resources, significant time and budget in identifying qualified investigators and setting up study sites

**Objective:** To create a shared repository of investigators

**Benefits:** Reduced cost and time of setting-up and running clinical trials
**Pediatric Trial Efficiencies**

**Unmet Need:** There are often an insufficient number of pediatric patients accessible to sponsors that can participate in clinical trials within the required timeframe.

**Objective:** Lead the development and implementation of solutions which improve the operative feasibility and conduct of pediatric clinical trials.

**Benefits:** Faster access to new drugs for pediatric patients, reduce burden on patient, faster and more efficient site contracting and reduced trial costs.
Clinical Trial Diversification

**Unmet Need:** Ethnic minority representation in clinical trial populations is often not reflective of the prevalence of the disease being treated.

**Objective:** To enhance the ability of sponsors and sites to achieve representative diversity within clinical trial populations.

**Benefits:** Broader understanding of drug safety and effectiveness. Increased ability to access, recruit and retain patients in clinical trials. Greater number of effective and high quality minority serving sites.
Clinical Data Transparency

**Unmet Need:** With increased transparency, there is a need to standardize the approach to protecting the privacy of individuals involved in clinical trials.

**Objective:** Develop a consistent approach for redacting privacy information found in clinical study reports and an approach for the anonymization of patient level data shared with the broader healthcare community.

**Benefits:** Enhance transparency and facilitate future research preserving the privacy of patients, investigators and clinical trial staff for operational transparency issues related to privacy.
Unmet Need: Clinical QMS requirements are fragmented across multiple guidances contributing to quality issues

Objective: Identify ways to improve quality industry-wide through partnerships with regulatory agencies and other industry stakeholders

Benefits: Enhanced patient safety by improved quality, assure data integrity, minimize delays in clinical trials, and bring drugs to market more quickly