TransCelerate
BIOPHARMA INC.

ACCELERATING THE DEVELOPMENT OF NEW MEDICINES

*Efficiency Enhancing Collaboration in Clinical Trials*

Grzegorz Maciążka,

 Dyrektor Badań Klinicznych, GSK, Związek Pracodawców

Innowacyjnych Firm Farmaceutycznych INFARMA

# Pharmaceutical R&D leaders identified collaboration as a key opportunity for generating industry-wide efficiencies

## Conducted an industry survey on areas amenable to collaboration
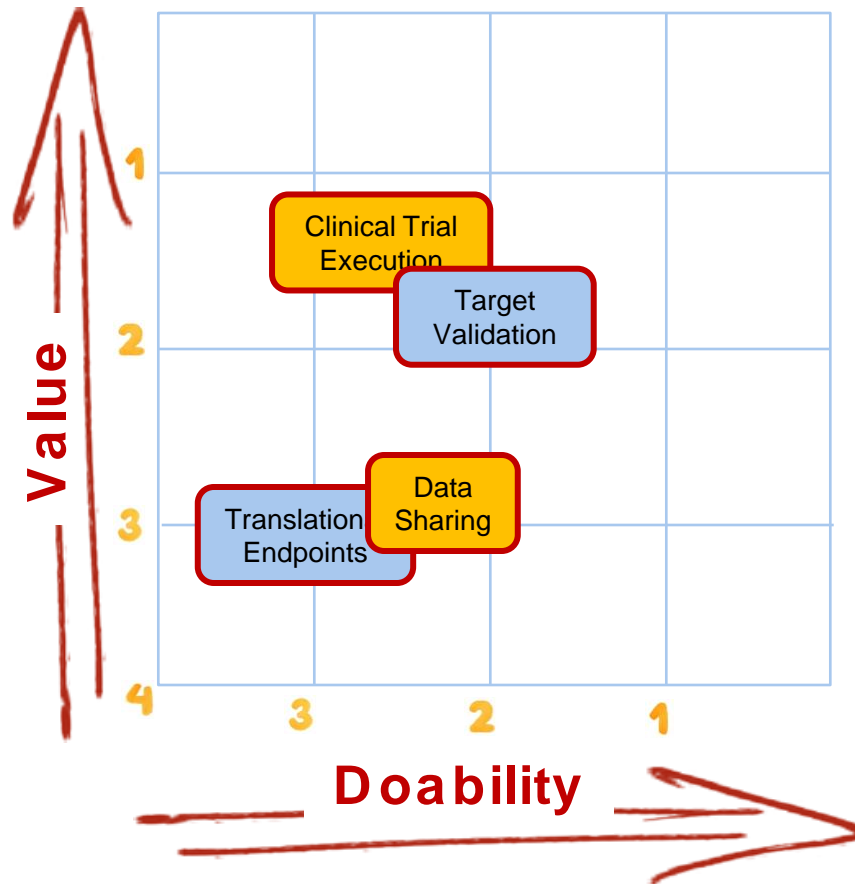
# Not for profit entity created to drive collaboration as means to developing solutions for overcoming inefficiencies

## Our vision

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

## Our mission

To collaborate across the global research and development community to identify, prioritize, design and implement solutions that drive the efficient, effective and high quality delivery of innovative new therapies.

## Our core values

- Quality
- Transparency & Openness
- Trust & Integrity
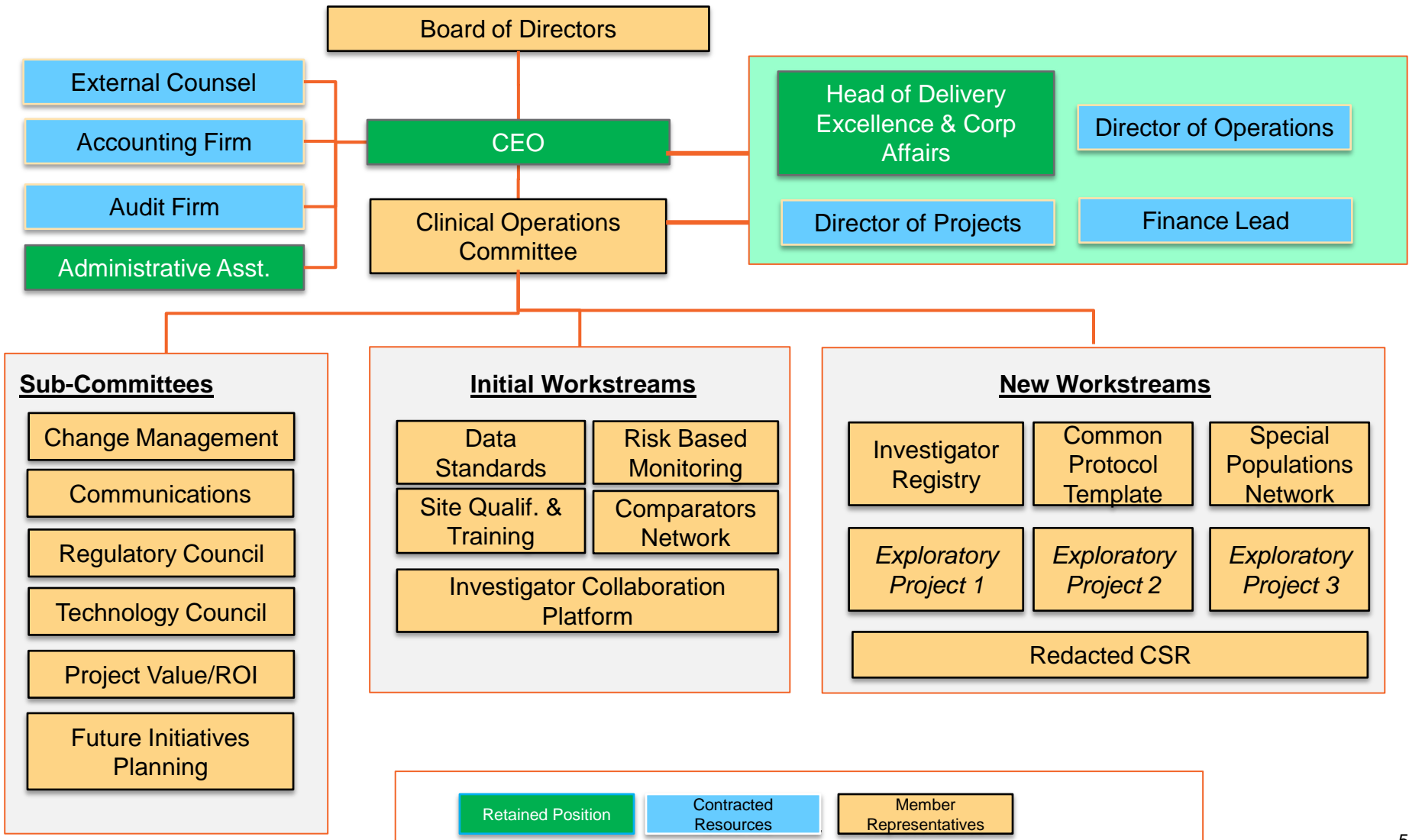- Collaboration
- Courage

# An Entity that Engages with the Wider Clinical Ecosystem Globally

**Strategically focusing engagement efforts with selected key stakeholder groups**
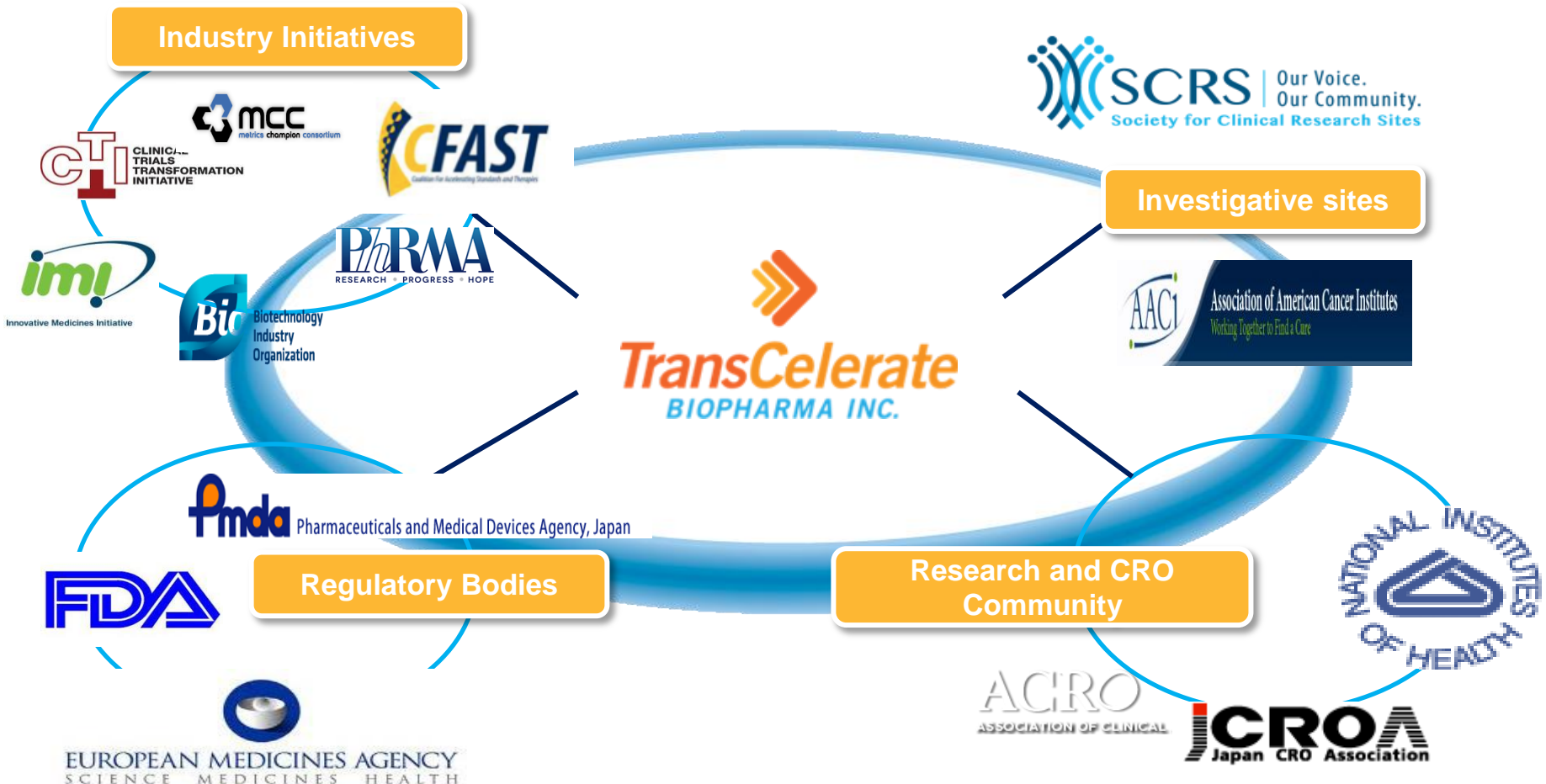


*The intent is not to recreate, but partner whenever feasible*

# A flat organization structure has been developed to manage projects and operational activities



**Board of Directors**

- External Counsel
- Accounting Firm
- Audit Firm
- Administrative Asst.

**CEO**

**Clinical Operations Committee**

- Head of Delivery Excellence & Corp Affairs
- Director of Operations
- Director of Projects
- Finance Lead

**Sub-Committees**
- Change Management
- Communications
- Regulatory Council
- Technology Council
- Project Value/ROI
- Future Initiatives Planning

**Initial Workstreams**
- Data Standards
- Risk Based Monitoring
- Site Qualif. & Training
- Comparators Network
- Investigator Collaboration Platform

**New Workstreams**
- Investigator Registry
- Common Protocol Template
- Special Populations Network
- *Exploratory Project 1*
- *Exploratory Project 2*
- *Exploratory Project 3*
- Redacted CSR

Legend:
- Retained Position
- Contracted Resources
- Member Representatives

# The Charter Members of TransCelerate Include Major Pharmaceutical Companies

**Annalisa Jenkins (Board Chair)**

**TBA (Board Member)**

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

abbvie

**Paul Stoffels (Board Member)**
*Worldwide Chairman of J&J Pharmaceuticals*
**Martin Fitchet (Treasurer to the Board, Operations Committee)**
*Chief Operating Officer*

Johnson&Johnson

**Briggs Morrison (Board Member)**
*EVP, Global Medicines Development*
**Sue McHale (Operations Committee)**
*Executive Director, Global Project Delivery*

AstraZeneca

**Jan Lundberg (Board Member)**
*EVP of Science & Technology*
**Jeff Kasher (Operations Committee)**
*VP and COO Global Medical R&D*

Lilly

**Klaus Dugi (Board Member)**
*Corporate SVP, Medicines*
**Thor Voigt (Operations Committee)**
*Head of Global Clinical Ops, Biometrics & Data Management*

Boehringer Ingelheim

**John Hubbard (Board Member)**
*SVP Development Operations*
**Craig Lipset (Operations Committee)**
*Head of Clinical Innovation*

Pfizer

**Brian Daniels (Board Member)**
*SVP Global Development & Medical Affairs*
**Reb Tayyabkhan (Operations Committee)**
*Exec. Director, GDO Business Operations*

Bristol-Myers Squibb

**Corsee Sanders (Board Member)**
*Global Head of Development Innov. & Clin Ops*
**Heather Cox (Operations Committee)**
*Global Head, CNS & Metabolics TA*

Roche

**Patrick Vallance (Board Member)**
*President, Pharmaceuticals R&D*
**Lynn Marks (Corporate Secretary)**
*SVP, Clinical Platforms & Sciences*
**Pete Milligan (Operations Committee)**
*VP, Clinical Platforms Transformation*

gsk GlaxoSmithKline

**Elias Zerhouni (Board Member)**
*President of Global R&D*
**Andy Lee (Operations Committee)**
*SVP, Head Global Clinical Operations*

SANOFI

# Nine new member companies joined TransCelerate in 2013

## Board Members

**Peter Carberry (Board Member)**
*SVP & Head of Global Development Operations*
**Nancy Sacco (Operations Committee)**
*Executive Director, Development Sciences/Strategic*

**Alfred Sandrock (Board Member)**
*SVP, Head of Development Sciences & CMO*
**Murray Abramson (Operations Committee)**
*VP, Global Clinical Operations*

**Steve Gilman (Board Member)**
*EVP, R&D and CSO*
**Uschi Stoutenburgh (Operations Committee)**
*Senior Director, Clinical Operations*

**TBA (Board Member)**

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

**Marco Taglietti (Board Member)**
*President, Forest Research Institiute & CMO*
**Ulo Palm (Operations Committee)**
*SVP, Clinical Operations & Biometrics*

**Garry Neil (Board Member)**
*Global Head, R&D*

## Non-Board Members

**Steve Johnson (Operations Committee)**
*SVP, R&D Business Services*

**Gareth Morgan (Operations Committee)**
*SVP, Portfolio Management, Global Development Office*

**Jonathan Zung (Chairman, Operations Committee)**
*VP, Global Development Operations*
**Brigitte Koch (Operations Committee)**
*VP, Head Global Clinical Project Management*

# Five opportunities were chosen for action based on industry readiness and ability to execute in 2013

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## *Prioritized Near Term Opportunities*

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

**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

**2** **Shared Site Qualification and Training**

**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

**3** **Common Investigator Site Portal**

**Objective:** Establish a single, intuitive interface for investigators use across the industry
**Benefits:** Ease of use and harmonized delivery of content and services for investigators

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

**Objective:** Accelerate current efforts underway through CDISC to establish efficacy data standards
**Benefits:** Increased quality of clinical data and enablement of industry end-to-end data flow

**5** **Comparator Drugs for Clinical Trials**

**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

**Projects have the shared goals of increased quality, patient safety & accelerated development timelines**

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| Five Selected Areas of Focus Have the Shared Goals of Increased Quality, Patient Safety and Accelerated Development Timelines | | | |
|---|---|---|---|
| Initiative | Objective | Benefit | • Progress to date |
| **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 | • Partnered with CDISC, Critical Path Institute, FDA and NCI on CFAST Therapeutic Area Program Steering Committee<br>• Asthma, Diabetes, Cardiovascular endpoints, QT studies, Multiple Sclerosis, Hepatitis C and Traumatic Brain Injury selected as first TAs of focus<br>• Approved Breast Cancer, Lipid-Lowering, COPD, CV Imaging, MDD, RA, Psoriasis & Schizophrenia project proposals<br>• SHARE environment (metadata repository) press release issued with CDISC in June 2013; SHARE R1 Release went live Jan 31 2014<br>• Asthma Therapeutic Area User Guide V1.0 published Nov 27 |
| **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<br>• Reduce the chance of counterfeit drug in study supply chain<br>• Share critical data – like solid dose ambient temp excursions | • Determined in-scope products and required documentation for distribution model<br>• Defined principles and process for drug distribution model<br>• MSA's between members finalized<br>• First set of transactions initiated in July 2013<br>• Press release issued in August 2013<br>• Multiple transactions continue to occur and direct benefits being realized<br>• Expansion of network activities for 2014 |

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|---|---|---|---|
| **Initiative** | **Objective** | **Benefit** | **Progress to date** |
| **Model Approach for High-Quality, Risk-Based Monitoring** | Develop an industry framework for targeted, risk based clinical trial monitoring | • Improvement in data quality and patient safety for clinical trials<br>• Reduction in effort expended on low-value activities | • FDA and EMA feedback incorporated<br>• Position paper and methodology published in May 2013 for access to entire clinical trial community;<br>  ➢ Update to the paper and associated training materials published in January 2014<br>• More than 30 pilots identified and launched with 8 of these reviewed by FDA prior to initiation<br>• Over 2000 unique downloads of position paper<br>• Conducted FDA orientation for the TransCelerate RBM methodology |
| **Shared Site Qualification and Training** | Mutual recognition of GCP training between pharmaceutical companies and streamlining site qualification process | • Improved quality of clinical sites and accelerated study start-up times<br>• Reduce site burden | • Establishment of framework for mutual recognition of GCP training completed<br>  ➢ Minimum content elements<br>  ➢ Process for awarding certificates<br>  ➢ Process for training providers to self attest to minimum criteria/courses<br>• Team formed to streamline site documentation<br>• Mutual recognition framework established among members<br>• Press release issued in June 2013<br>• Project scope increased in November 2013<br>• Created and published to TransCelerate website:<br>  ➢ CV and Site Profile Documents and Guidance<br>  ➢ Guidance for completion of FDA document 1571/72<br>  ➢ Guidance and Delegation of Responsibility form<br>• Planning for PI Oversight Training and Training for |

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|---|---|---|---|
| **Initiative** | **Objective** | **Benefit** | **Progress to date** |
| **Shared Site Collaboration Platform (formerly Common Investigator Site Portal)** | Establish a single, intuitive interface for investigators, regulators and member companies | • Ease of use and harmonized retrieval of content and services for investigators<br>• Reduce site burden<br>• Reduce member company costs | • Defined components to leverage from existing industry portals<br>• Board approval for analysis phase in June<br>• Systems integrator, product partner and hosting partner selected<br>• Early adopters of system confirmed |

# The future - a roadmap was created with the future state in mind

## Current State

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

## Roadmap
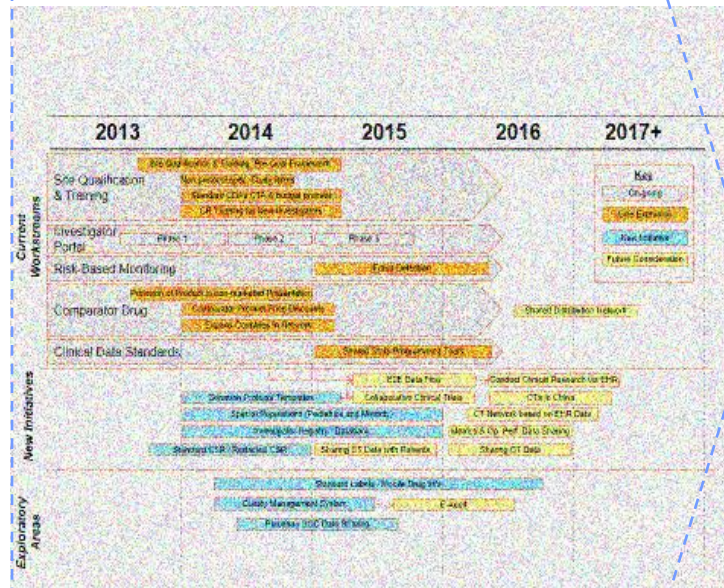


## Future State

- Patient-centric clinical trial design
- End-to-end electronic data flow
- Seamless interfaces
- Automated
- Transparent
- Increased standardized solutions
- Less rework
- Quality by design
- Shorter cycle times
- Cost efficient
- Integration of Regulatory, Safety, and Medical Sciences
- Elimination of redundancies
- Conducting clinical trials together
- "Colossal Data Analytics"

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## Approved TransCelerate projects

**Ongoing projects**

- ➤ **Data Standards**
- ➤ **Risk Based Monitoring**
- ➤ **Comparator Network (ES)**
- ➤ **Site Qualification and Training (ES)**
- ➤ **Shared Site Collaboration Platform**

**New projects for 2014**

- ➤ *Redacted CSR* *
- ➤ **Common Protocol Template**
- ➤ **Investigator Registry**
- ➤ **Clinical Trial Networks**
    - ➤ **Pediatric patients**
    - ➤ **Minority patients**

**Exploratory projects for 2014**

- ➤ **Exploratory project I**
- ➤ **Exploratory project II**
- ➤ **Exploratory project III**

**(ES) – Expanded Scope for 2014**
*\* Workstream initiated to meet compliance deadline*

## Future State

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| Three New Initiatives Will Further Support the Goals of Increased Quality, Patient Safety and Accelerated Development Timelines | | | |
| --- | --- | --- | --- |
| **Initiative** | **Unmet Need** | **Description** | **Benefit** |
| **Common Protocol Template** | • Format of study protocols vary from company to company making interpretation difficult for study sites, IRBs, and regulators.<br><br>• Study protocols have become increasingly complex as no agreed upon standards exists driving up cost and time.<br><br>• Manual set-up of clinical systems based on non-standard "manual" protocols are time consuming, costly, and prone to error | Standardize the format of clinical protocols to ease interpretation & enable down-stream automation of many clinical processes. Develop industry-wide & regulator accepted standards for required protocol endpoints | • Higher productivity of sponsors, sites, IRBs, and regulators<br><br>• Less costly and time consuming clinical trials<br><br>• Enabler for downstream automated setup of clinical and operational systems & disclosure activities |

| Three New Initiatives Will Further Support the Goals of Increased Quality, Patient Safety and Accelerated Development Timelines | | | |
|---|---|---|---|
| **Initiative** | **Unmet Need** | **Description** | **Benefit** |
| **Investigator Registry** | Sponsors invest significant time and money in identifying qualified investigators and setting up study sites | To create a shared repository of investigators to support targeted patient selection | • Reduced cost and time of setting-up and running clinical trials |
| **Special Populations Clinical Trial Networks (minority & pediatrics)** | • Qualified investigators with adequate study patients are difficult to find for special populations – e.g. pediatric and minority<br><br>• Studies in minorities and pediatrics are costly and lengthy<br><br>• Efforts are put into repeatedly establishing a network for a single study only to disperse the network after study completion | Lead the development of global investigator networks for pediatric and minority populations including governance, investigator and patient registries, and technical infrastructure | • Faster development of new drugs in both pediatric and minority populations<br><br>• Reduced costs of pediatric and minority trials |

# Key Accomplishments to Date

| Top Accomplishments in first year |
|---|
| 1 | Mobilized 10 companies to create TransCelerate, 10 new members joined in 2013 |
| 2 | Created a lean and functional infrastructure of a not for profit entity |
| 3 | Initiated pilots, published SHARE environment and asthma standard released |
| 4 | Published the criteria for mutual recognition of GCP training |
| 5 | Published the framework and approach for risk based monitoring |
| 6 | Launched pilot studies for RBM across multiple member companies and TAs |
| 7 | Engaged multiple organizations – CTTI, SCRS, BIO, IOM, NIH, ACRO, IMI etc |
| 8 | First transaction of comparator drugs among member companies initiated |

| Key Upcoming Milestones |
|---|
| 1 | Initiate new projects and expand scope on some existing projects |
| 2 | Continue to engage key regulatory agencies (EMA, FDA, CFDA & PMDA) |
| 3 | Continue robust engagement of other key stakeholders |
| 4 | Develop operational models & sustainability plans for projects |
| 5 | Launch first version of Shared Site Collaboration Platform in 2014 |

# Stan wdrożenia inicjatyw w Polsce

| | |
|---|---|
| **TransCelerate GCP Training Certificate** |  |
| **Template for investigator Curriculum Vitae (CV)** |  |
| **Risk Based Monitoring** |  |

**http://www.transceleratebiopharmainc.com/**