TransCelerate BIOPHARMA INC.

eSource

Workstream Overview

October 2019

TransCelerate:

A Not-for-Profit Entity Created to Foster Collaboration

Our Shared Vision:

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

Current state of organization

2012

TransCelerate Founded

10 MEMBER COMPANIES

5 INITIAL INITIATIVES

2016

BioCelerate Founded

BioCelerate

focus on preclinical research

Today

20 MEMBER COMPANIES

Regeneron most recent member

25+ INITIATIVES

including 4 pharmacovigilance initiatives

BREADTH & DEPTH

Over 30 solutions being delivered across 25+ initiatives, across 3 strategic priorities

CULTURE OF COLLABORATION

With an effective and proven governance structure have increased the ease and desire to collaborate

ENABLING PLATFORM TRIALS

12+ initiatives deliver solutions that enable future platform trials

DataCelerate

platform to enable data sharing

The Reach of our Global Membership is Expanding

Membership is available to biopharmaceutical research and development organizations that engage in innovative discovery, development and manufacturing of new medicines*.

abbvie
Allergan
AMGEN
astellas
AstraZeneca
Boehringer Ingelheim
Bristol-Myers Squibb
EMD SERONO
MERCK
gsk GlaxoSmithKline
Johnson & Johnson
Lilly
MERCK & CO., INC. Kenilworth, N.J., U.S.A.
novo nordisk
NOVARTIS
Pfizer
REGENERON SCIENCE TO MEDICINE
Roche
SANOFI
SHIONOGI
ucb

There are over **1,000** people from Member Companies that design and develop TransCelerate solutions.

* to be eligible for membership, companies must meet specified eligibility criteria.

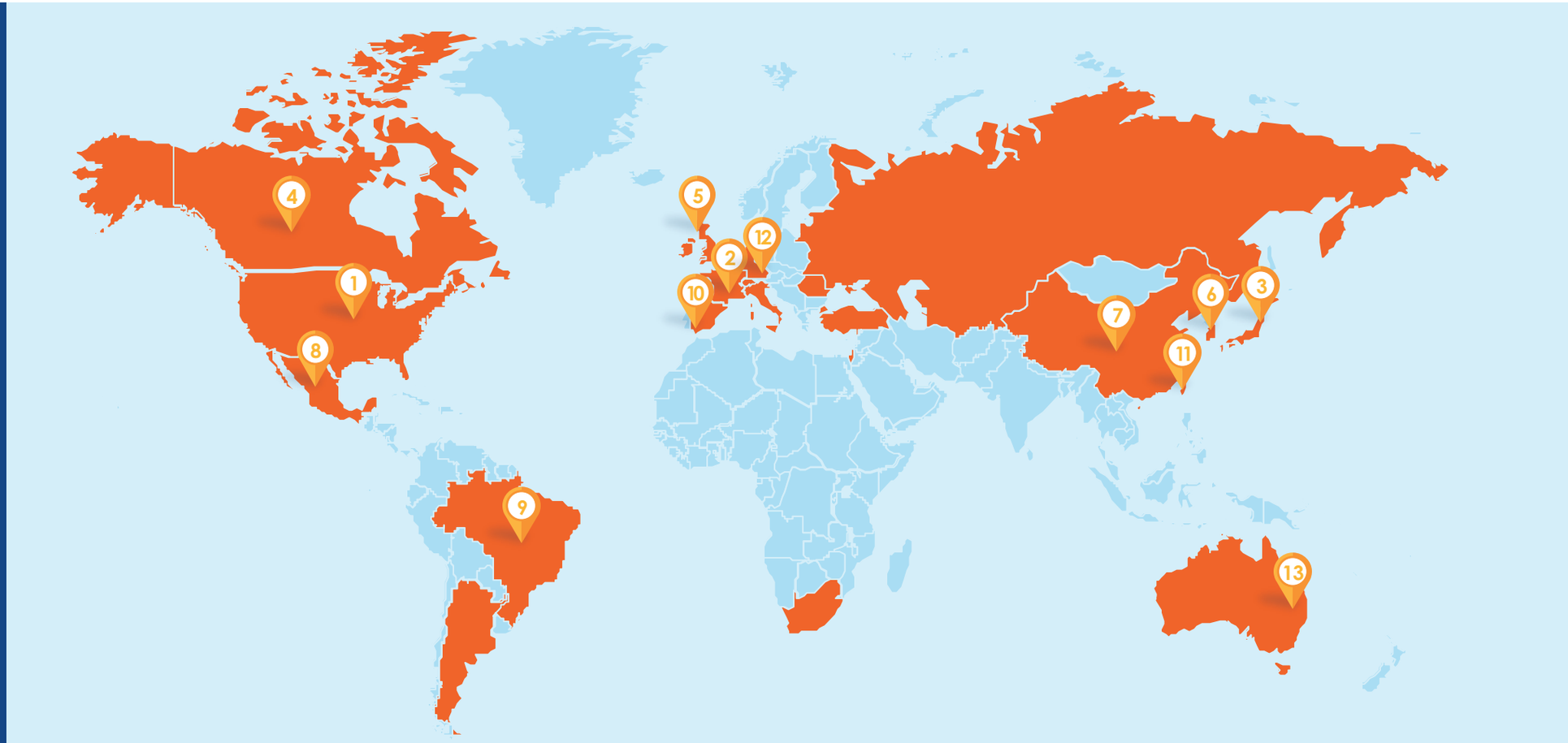
Our Presence, Impact and Engagement is Worldwide

Our Country Network spans

22
COUNTRIES,
and

13 **GLOBAL REGULATORY AUTHORITIES**

have engaged with TransCelerate.



1 FDA
2 EMA
3 PMDA
4 Health Canada
5 MHRA
6 MFDS
7 CFDA
8 COFEPRIS
9 ANVISA
10 AEMPS
11 TFDA
12 BfArM
13 TGA

External Collaboration will continue to play a critical role in achieving our future state

TransCelerate BioPharma and FDA/NIH

COLLABORATE

on Aligned Common Protocol Template

Society for Clinical Research Sites Announces TransCelerate BioPharma's Ongoing

COMMITMENT TO PARTNERSHIP

With Scrs Site Advocacy Groups

CDISC and TransCelerate Announce New Standard for Breast Cancer to

SUPPORT DATA SHARING for Oncology Research

As a single stakeholder organization, we understand the value of robust collaboration with key stakeholders* across the R&D ecosystem which provide unique and important insights and perspectives.

INVESTIGATOR SITES*

ACRP · AACI Association of American Cancer Institutes · SCRS Our Voice. Our Community. Society for Clinical Research Sites

RESEARCH AND CRO COMMUNITY*

ACRO Association of Clinical · JCROA Japan CRO Association · NIH National Institutes of Health Turning Discovery Into Health · CROForum

PATIENT ADVOCACY GROUPS*

CISCRP · International Alliance of Patients' Organizations

OTHER ASSOCIATIONS*

Bio Biotechnology Industry Organization · CDISC · Clinical Trials Transformation Initiative · DIA · efpia · PhRMA Research · Progress · Hope · imi

HEALTH AUTHORITIES*

FDA · European Medicines Agency Science · Medicines · Health · Health Canada · Pmda Japan

* Representative organizations, not exhaustive

TransCelerate's Initiatives deliver practical solutions to overcome inefficiencies in research & development

OUR MISSION:

Collaborate across the global biopharmaceutical R&D community to identify, prioritize, design and facilitate implementation of solutions designed to drive the efficient, effective and high-quality delivery of new medicines

HARMONIZE PROCESS AND SHARE INFORMATION

- Clinical Data Standards
- Common Protocol Template
- **Common Statistical Analysis Plan Template***
- Comparator Network
- DataCelerate™
- eSource
- **Digital Data Flow***
- Investigator Registry
- Placebo Standard of Care
- Toxicology Data Sharing

IMPROVE THE PATIENT AND SITE EXPERIENCE

- Clinical Research Access and Information Exchange
- Clinical Research Awareness
- eConsent
- eLabels
- Patient Experience
- Patient Technology
- Site Qualification and Training
- Shared Investigator Platform

ENHANCE SPONSOR EFFICIENCIES & DRUG SAFETY

- **Advancing Safety Analytics***
- Clinical Data Transparency
- **Data Monitoring Committee***
- **Intelligent Automation Opportunities in Pharmacovigilance***
- Interpretation of Pharmacovigilance Regulations
- **Protocol Deviations***
- Quality Management System
- Risk-Based Monitoring
- Value of Safety Information Data Sources

*** New Initiative launched in 2018**

eSource Overview

What is eSource?

Currently, there is no standard industry definition of eSource

Per FDA, data initially recorded in electronic format

FDA also notes that eSource data "can include information in original records and certified copies of original records of clinical findings, observations, or other activities captured prior to or during a clinical investigation used for reconstructing and evaluating the investigation"

TransCelerate describes eSource and its four different modalities as…

"Electronic source data are data initially recorded in electronic format…"

Non-CRF
The collection and transfer of electronic data from internal sponsor sources or external vendors into data repositories/warehouses without into a Case Report Form (CRF).

Devices and Apps
The collection and management of clinical data from non-site personnel, wearables, and sensors.

DDC
The direct entry of clinical data by site staff into a mobile application or EDC system.

EHR
The collection and reuse of data for use in clinical research from site/patient electronic health record systems

**The eSource team recognizes that some technologies cross these boundaries and that these categories will likely evolve over time due to technological advances*

eSource adoption faces multiple challenges

Lack of change management

Acceptance of novel technology solutions that enable eSource

&

Unknown quality and regulatory clarity on the data in these solutions

Lack of system interoperability

Interoperability is referred to by the Institute of Electrical and Electronics Engineers (IEEE) in basic terms as "the ability of a system or a product to work with other systems or products without special effort on the part of the customer. Interoperability is made possible by the implementation of standards."

Concerns over eSource acceptability as endpoints; lack of clarity from key study stakeholders

Low risk tolerance in Pharma industry

However, the benefits of an eSource Investment impacts all stakeholders

Value	Value Drivers		
	Time	Cost	Quality
For **SPONSORS**, harmonized clinical data collections standards resulting in easy of data exchange between different sources and reduced clinical development timelines			
For **SITES,** reduces the manual transcription of data, related errors, and completeness of data through the utilization of existing data thus free up site to focus on the patient			
For **PATIENTS,** potential reduction in site visits due to continuous data collection through eSource modalities			
For **REGULATORS**, greater traceability for end-to-end data flow for improved Health Authority review & approval			

TransCelerate eSource Workstream

The eSource Initiative works towards the advancement of the digitalization of clinical development for patients, sites, and sponsors

2015
Workstream founded

2016
Understand and Align

Uncovered the common aspirations to modernize the data collection

Identified Member Company priorities in adoption of eSource Modalities

Awareness and Action

Revealed technology vendor willingness to engage with pharma

Exposed primary barrier to eSource advancement as a people and process issue

Prompted CDISC mapping to FHIR and EDC Vendors to participate in HL7 FHIR connectathon work

2017

2018
Focus and Initiate

Initiated TransCelerate directed mapping to FHIR

Connected Duke with Member Companies for EHR to EDC Pilot

Identified need of site training and understanding of eSource capabilities

Recommended pharma as a trusted entity in the exchange framework to enable scalable EHR

2019
Engage and Accelerate

Engage with global regulatory agencies

Accelerate the maturity of EHR as an eSource for Clinical Research to create a scalable solution

Publish Sponsor call to action for change

Describe pharma needs to vendors through Logical Architecture and Data flow Diagram in collaboration with Tech council

Future State Interoperability: Explosion of FHIR

Pharma companies have yet to invest significate resources to solve the EHR interoperability issue

Changes in policy, emerging technologies, and advances in EHR data are transforming healthcare

These advances have the potential to provide great value to both patient care and Clinical Research

Amazon, Google, IBM, Microsoft, Oracle and Salesforce pledge to remove interoperability barriers

At the White House, technology heavy
work together improving data exchan
the Argonaut Project and more.

https://www.healthcareitnews.com/news/amazon-g
salesforce-pledge-remove-interoperability-barriers

A FHIR Future Burns Brightly for Population Health Management

As the healthcare industry warms up to FHIR, population health management and health data interoperability may be in for some significant improvements.

https://healthitanalytics.com/features/a-fhir-future-burns-brightly-for-population-health-management

SMART on FHIR app uses machine learning to help dermatologists make diagno

Physicians at one Missouri health system are using the
quickly reach conclusions and move forward with the b
care options, its CMIO says.

http://www.healthcareitnews.com/news/smart-fhir-app-uses-machi
learning-help-dermatologists-make-diagnoses

UPDATE
JANUARY 24, 2018

Apple announces effortless solution bringing health records to iPhone

Health Records Brings Together Hospitals, Clinics and the Existing Health App to Give a Fuller Snapshot of Health

https://www.apple.com/newsroom/2018/01/apple-announces-effortless-solution-bringing-health-records-to-iPhone/

eSource Initiative, to date and beyond

Various eSource Assets and Industry Resources can be found at the eSource Website (Link to eSource Assets Page)

Knowledge Insights

- **eSource Sponsor Landscape** *(published)*
- **eSource Technical Landscape** *(published)*
- **Best Practices for non-CRF Data**
- **Roadmap to eSource Adoption: A TransCelerate Perspective** *(pending publication)*
- **Technology Considerations for the Future State** *(pending publication)*

Tools & Solutions

- **eSource Site Capability Questionnaire**
- **CDISC Lab Semantics in FHIR Implementation Guide**
- **eSource Site Maturity Curve** *(pending)*
- **Method to calculate value & cost on eSource adoption** *(pending)*
- **Regulatory Landscape Assessment** *(pending)*

- **Engage with Site Advocacy Groups**
- **Collaborate with Standards Setting Organizations (e.g. HL7)**
- **Share and align information at industry events and conferences**