TransCelerate BIOPHARMA INC.

TransCelerate Overview

DCRF Jaarcongres 2019
TransCelerate Workshop
Mirjam Steinbuch

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

ENHANCING INDUSTRY COLLABORATION

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

FACILITATING FUTURE PLATFORM TRIALS

12+ initiatives deliver solutions that facilitate 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
Merck KGaA
Darmstadt, Germany
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

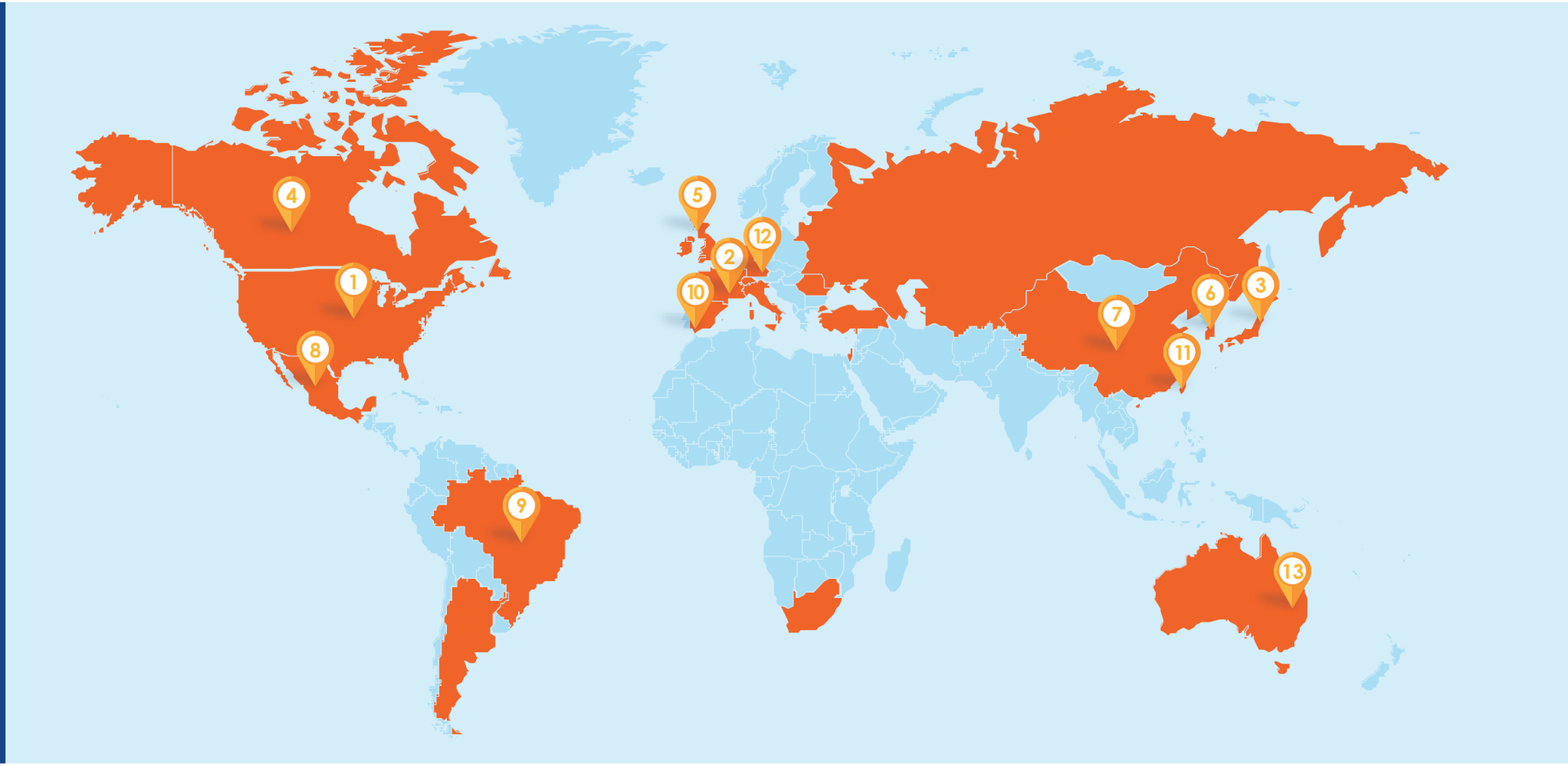
Our Country Network spans

30 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

FOCUS ON SITES

by launching 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 Association of Clinical Research Professionals

AACI Association of American Cancer Institutes

SCRS Our Voice. Our Community. Society for Clinical Research Sites

RESEARCH AND CRO COMMUNITY*

ACRO Association of Clinical Research Organizations

JCROA Japan CRO Association

NIH National Institutes of Health Turning Discovery Into Health

CROForum

PATIENT ADVOCACY GROUPS*

CISCRP The Center for Information Study on Clinical Research Participation

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
- Comparator Network
- DataCelerate®
- eSource
- Digital Data Flow
- Placebo Standard of Care
- Toxicology Data Sharing
- **Common Clinical SAE***

IMPROVE THE PATIENT AND SITE EXPERIENCE

- Clinical Research Access and Information Exchange
- Clinical Research Awareness
- eConsent
- eLabels
- Investigator Registry
- 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 Guidance and Regulations***
- **Modernization of Statistical Analysis***
- Protocol Deviations
- Quality Management System
- Risk-Based Monitoring
- Value of Safety Information Data Sources

*** New Work approved by TransCelerate Board for 2019**

Our Work in Preclinical Development: BioCelerate

BioCelerate, a subsidiary of TransCelerate BioPharma, focuses on the identification and development of pragmatic and tangible solutions to improve efficiencies in preclinical research.

Leadership and Governance Structure: BioCelerate has been established as a separate legal subsidiary of TransCelerate with separate funding and support.

Toxicology and Background Control Data Sharing Initiative

The first Initiative, **Toxicology Data Sharing**, is working to help close critical gaps between patient response and preclinical toxicology findings.

Nonclinical Study Optimization

The initiative is focused on working with key stakeholders to implement common best practices such as harmonization of SEND data sets and authoring of protocols and reports.

Membership in TransCelerate is a prerequisite for BioCelerate membership.

Our Work in Data Sharing: DataCelerate

DataCelerate will be used to aggregate and analyze preclinical and clinical information* to improve drug development efficiency and bring new medicines to patients faster.

Toxicology & Background Control Data
May, 2018

Enables participating companies to make data-driven decisions on compound progression based on an increased understanding of on-target and off-target toxicity.

Placebo Standard of Care Data
2019

Enables participating TransCelerate members to collect and share anonymized clinical data historically gathered in the placebo and standard-of-care arms of clinical trials among participating member companies.

Additional data types currently under assessment

Our Solutions provide transformational value and impact

SPEED

"Implementation of the GCP mutual recognition was an important win for us Now it is one less training that the PIs have to complete, **which speeds up our startup process**…"

- Sponsor Company on Site Qualification & Training

COST

"Given the intense price pressure pharma is under, **we need to get inefficiency out of trials** to make them economical. Ultimately that is how the market will grow"

- CRO on TransCelerate's industry impact

QUALITY

"We're seeing improvement in quality…[Risk Based Monitoring] **allowed us to improve patient safety and data integrity.**"

– Sponsor Company on Risk Based Monitoring

EXPERIENCE

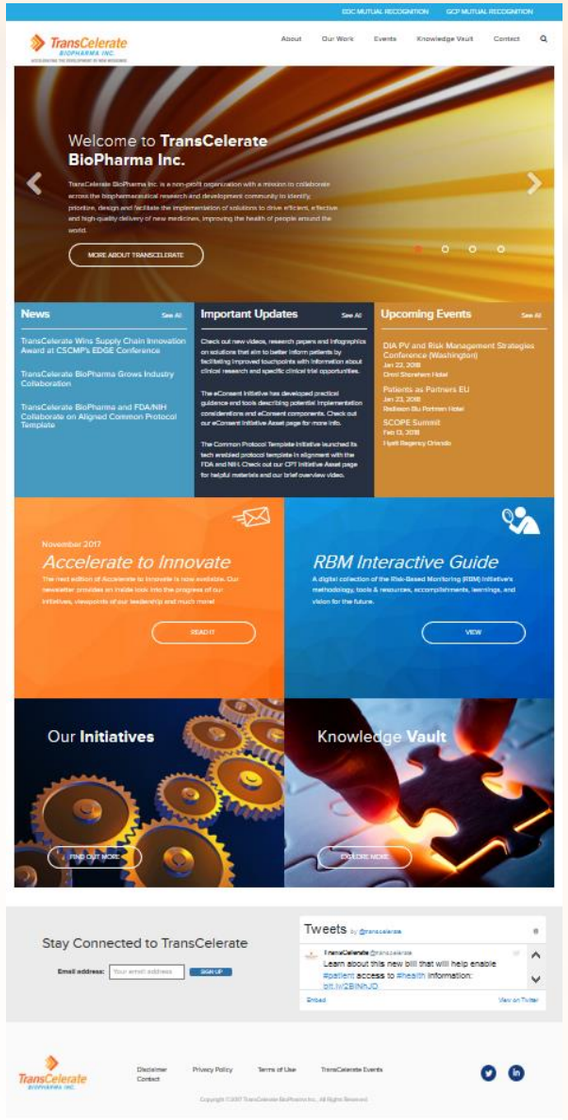
"Stability data information helps support temperature excursions that may occur during shipping or storage… [allowing] **use of product** that would've normally been discarded."

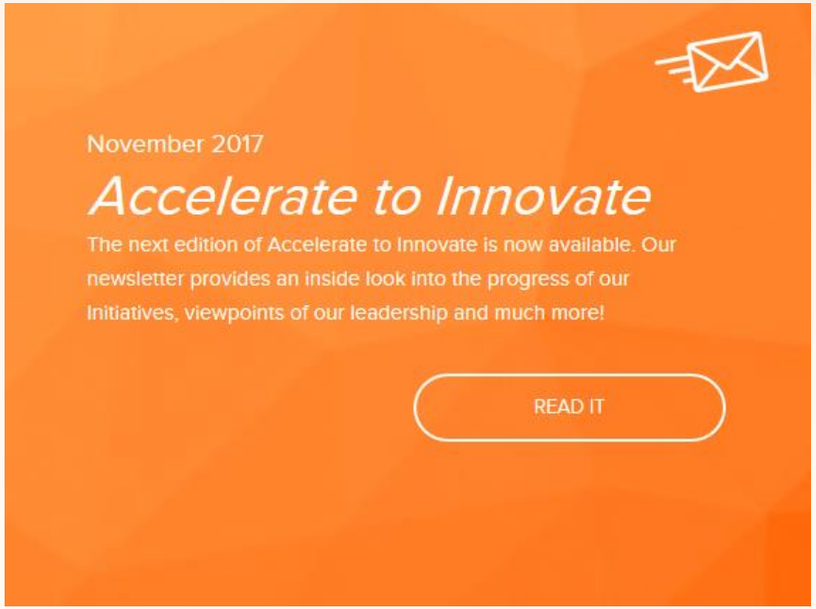
– Sponsor Company on how the Comparator Network reduces study delays

MINDSET

"Harmonizing our templates worked out well for both sides. Developing a template with FDA & NIH alone, **there is no way that we would have found traction with industry**."

– Health Authority on the Common Protocol Template





Visit us, for more information:
www.TransCelerateBioPharmaInc.com

Watch our "About Us" Video

Sign up for our Newsletter,
Accelerate to Innovate

@TransCelerate

TransCelerate BioPharma Inc.

Dutch Transcelerate Country Network

- Currently 12 representatives of member organisations
- Quarterly meetings
- Exchange news and experience
- Drive the key topics for NL

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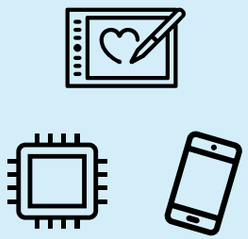
Patient technology

TransCelerate Patient Technology

How TransCelerate thinks about patient technology

Patient technology includes any digital technology with which patients interact to participate in clinical trial activities



TransCelerate believes that the use of patient technology in clinical trials has the potential to:

- enhance patient experience and engagement
- streamline clinical trial processes, or
- enable better, more robust data collection in clinical trials.

TransCelerate Patient Technology

Scope of Activity and Focus Areas

The TransCelerate Patient Technology Initiative is working to **enable** and **accelerate** clinical trial use of patient-facing digital technology, leading to richer, more objective data collection and enhanced patient experience.

Based on our research findings, we are focusing on the following areas:

CREATING TOOLS

Creating processes, methodology, or considerations towards the optimized implementation of patient technology

SHARING KNOWLEDGE

Sharing data and learnings between sponsor companies on PT implementation experience

IMPROVING REGULATORY CERTAINTY

Creating understanding between HAs and industry stakeholders on the use of PT (including NDEs)

TransCelerate Patient Technology

What We're Producing

Team Focus Areas

- Creating Tools
- Sharing Knowledge
- Improving Regulatory Certainty

Available!

Patient Technology Point of View White Paper

Published research describing the current landscape of patient-facing technologies (PT) in clinical research, including benefits, gaps, and opportunities

Available!*

Patient Technology Toolkit

Tools for industry stakeholders to support the implementation of Patient Facing Technologies.

*Implementation Framework & Regulatory Landscape Tool

Patient Technology Shared Insights Knowledgebase

Dynamic data repository for sharing Patient Technology experience via structured, searchable data on trials run by contributing sponsors

Novel Digital Endpoints Validation Blueprint (NDE)

Pathway for validating novel digital endpoints and novel methods of measuring existing endpoints, including health authority interaction

Regulatory Engagement

Creating understanding and dialogue between HAs and industry stakeholders on the use of PT

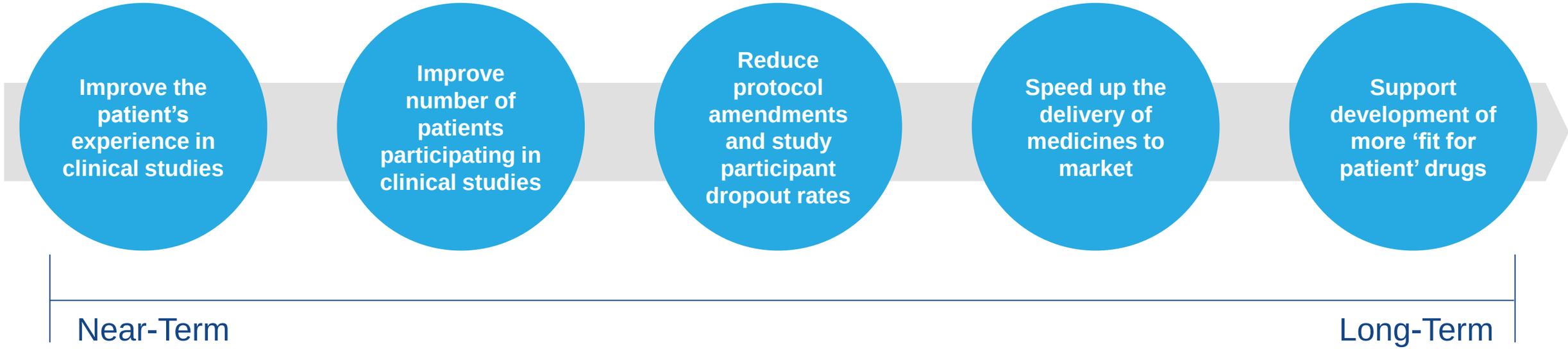
External Engagement

Enriching our work products through engagement with industry stakeholders

Patient centricity

The Impact of Engaging Patients in the Design and Execution of Clinical Studies

- **By designing clinical studies with patient inputs, study sponsors can potentially…**

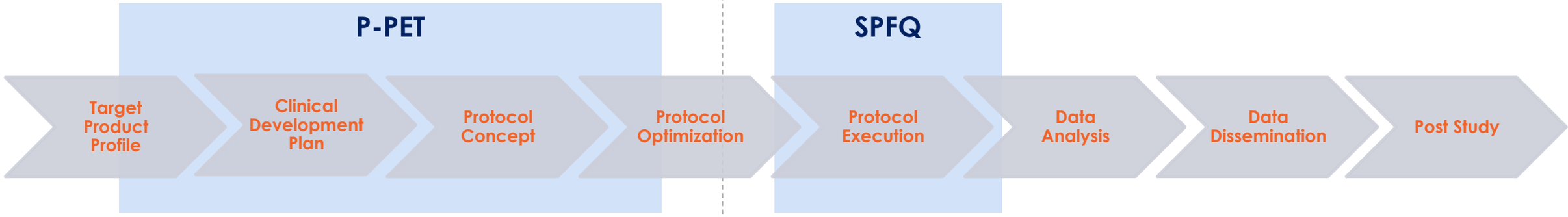


TransCelerate Patient Experience Initiative

- ***This initiative seeks to develop patient engagement tools will contribute to an improved partnership between sponsors and patients in clinical studies.***

Patient Protocol Engagement Toolkit

Study Participant Feedback Questionnaire Toolkit

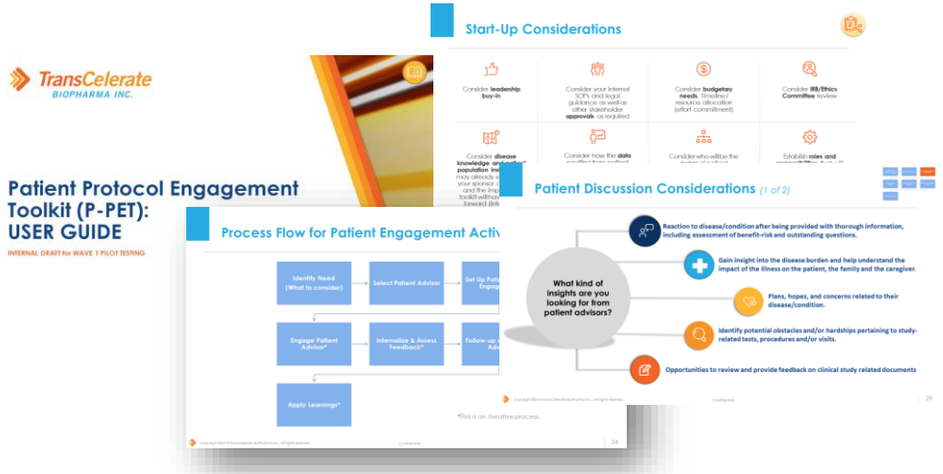


Design clinical studies with patient input

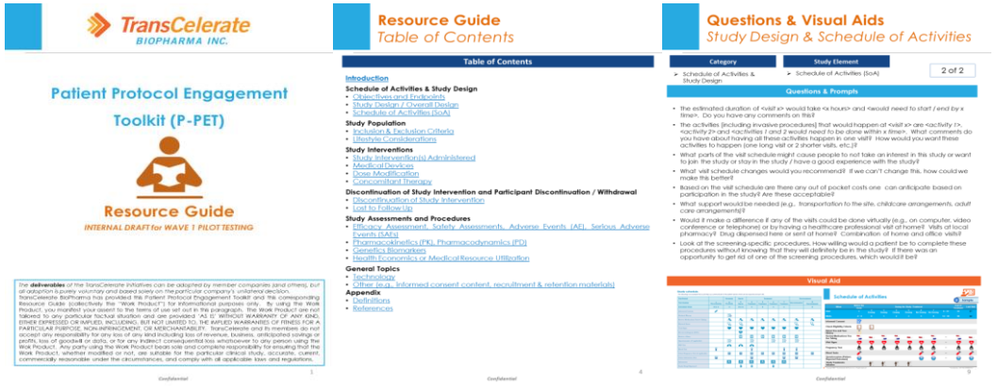
Gather patient feedback during clinical studies

Patient Protocol Engagement Toolkit (P-PET)

User Guide



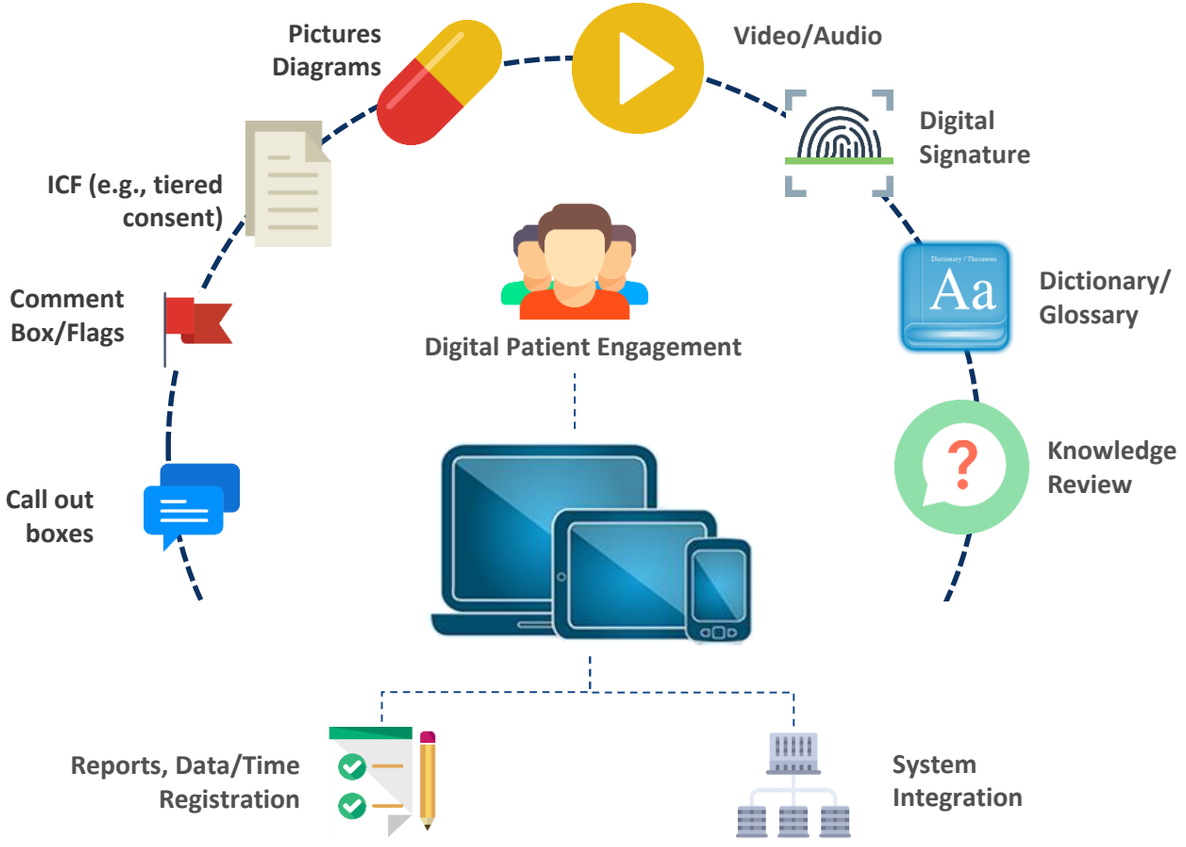
Resource Guide



Templates

E Consent

What is eConsent?



Empowers patients to make informed decisions through the use of interactive, multi-media components

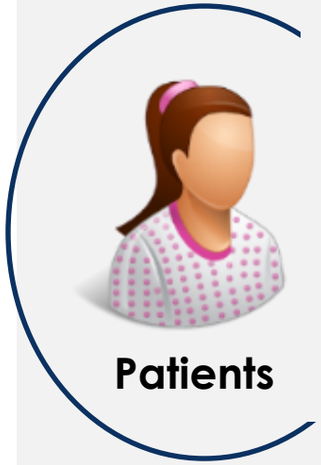
Enables the improved quality and efficiency of clinical trials through insight into the patient experience, improved data quality, and a fully electronic system.

eConsent is a tool which can improve the site staff/patient discussion

*The term patient refers to study participants, trial participants, subjects

eConsent Benefits

Patient-Focused Benefits



Patients

- ✓ Easy to understand
- ✓ Simplified and consistent
- ✓ Engaging and interactive
- ✓ No pressure/ at own pace
- ✓ Tailored to personal learning style

Health Authorities/Ethics

- Improved version control
- Improved protection of patients
- Fewer inspection findings
- Increased transparency

Sites

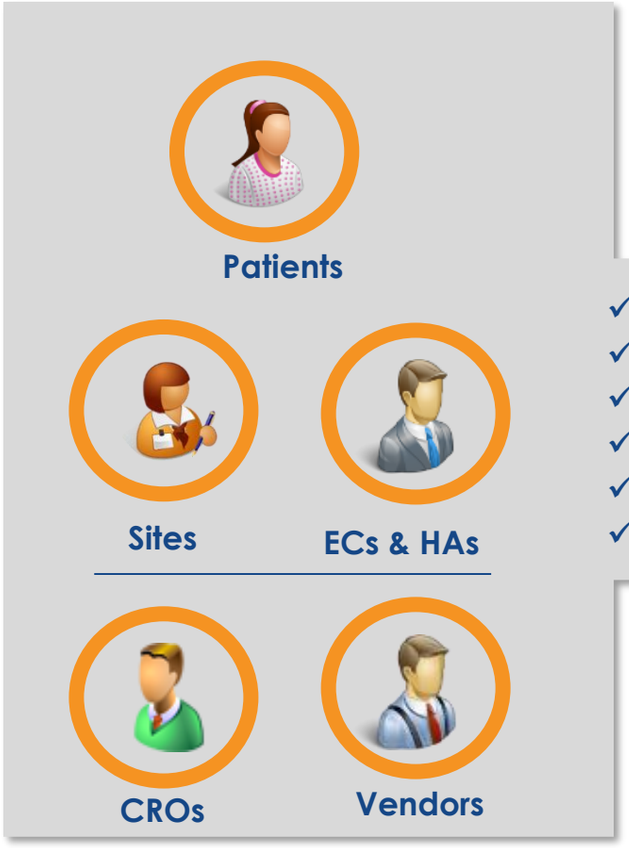
- Ability to focus on patient's targeted questions and areas of concern
- Less paper, less quality risk

Sponsors/CROs

- Timely insight into consent-related activities
- Continuous improvement of consent
- Improved efficiencies
- Improved ongoing oversight
- Potentially improve patient retention & compliance

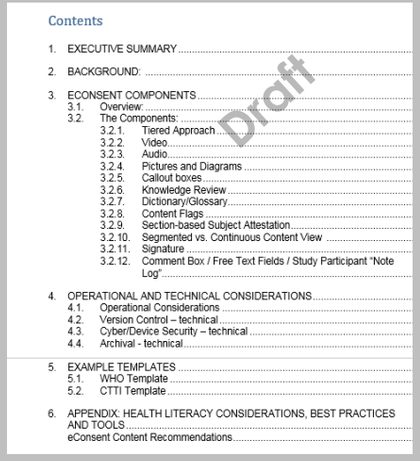
External Engagement focuses on receiving feedback on eConsent from involved stakeholders

Feedback received is being used to help develop our deliverables to ensure that we are taking into account the needs of all involved stakeholders.



- ✓ Focus Groups
- ✓ Market Research
- ✓ Meetings
- ✓ Other Initiatives
- ✓ Surveys
- ✓ Public Comment

Implementation Guidance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6. APPENDIX: HEALTH LITERACY CONSIDERATIONS, BEST PRACTICES AND TOOLS
   eConsent Content Recommendations

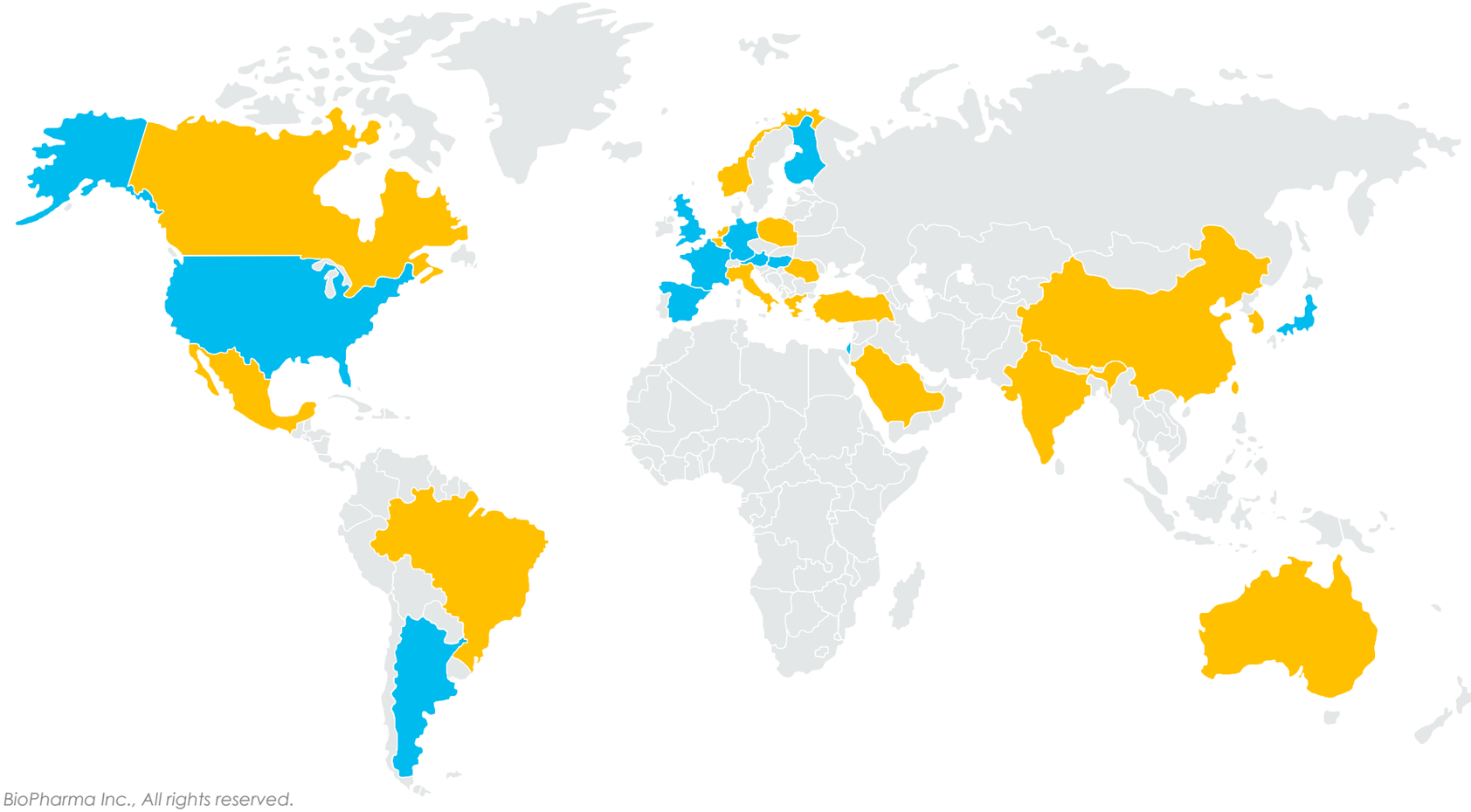
Draft

eConsent Around the World 2016

eConsent submitted

eConsent used to Consent patients

The following data was collected in December 2016 from 17 responses to the TransCelerate eConsent Landscape survey. The below global view represents the countries the 17 responders indicated where eConsent was submitted and of those submission countries, where eConsent was used to consent patients.



Protocol deviations

In 2018, TransCelerate launched an initiative to address industry-wide problems related to protocol deviations.

PROBLEM STATEMENT

The lack of clarity regarding the definition of “important” protocol deviations has led to both *over and under interpretation of deviations* which impacts the *planning, collection, analysis and reporting* of important protocol deviations.

IMPACT STATEMENT

Over and under interpretation of “important” protocol deviations could increase the noise in the PD management process, potentially:

- *Hindering* the identification of important patient safety information
- *Influencing* the reliability of trial results and human subject protection

Objectives of initiative

- Reduce noise to support rapid identification of *important* protocol deviations
- Increase process efficiencies at site and sponsor

BY

- Improving interpretation of guidelines, including definition
- Improving management of protocol deviation processes
- Engaging with the FDA and other Health Authorities on these critical issues and solutions.

With feedback from the agency, the team developed three tools to help clarify the definition and process for identifying an “important” PD.

PD PROCESS GUIDE

A proposed framework describing flexible PD management approaches, elements for consideration based upon proposed interpretation of the ICH E3 definition for important protocol deviations and other associated PD Guidance with links to the PD Process Map

PD MAP

A map of the PD management process containing processes for both important and non-important deviations

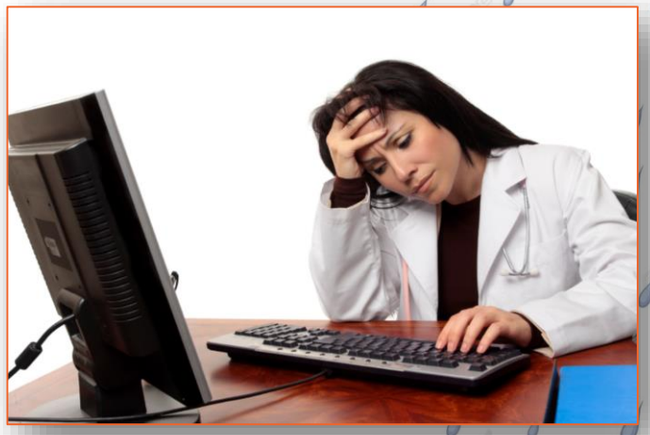
PD ASSESSMENT TOOL

A systematic template to assist in the identification and documentation of protocol specific ‘important’ deviations

Site Qualification and Training

Why did the Site Qualification & Training Initiative Start?

In 2012, Investigator Sites lived in a world filled with **duplicate training, forms and templates** causing **frustration, wasted time, and complexities.**



Site Qualification & Training Workstream & Solutions

Executive Summary

Vision

- Reduce **administrative burden** and **duplication of effort** for sites.
- **Support less experienced site** staff with resources to gain more information about clinical research.
- Foster stronger **partnerships** with sites.

Approach

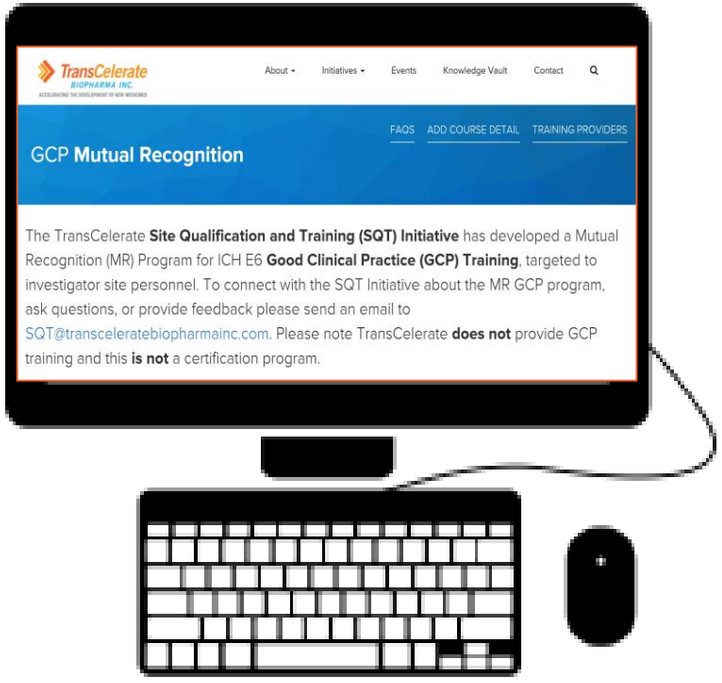
- Gain input from **external stakeholders.**
- Develop processes for **mutual recognition** of training.
- Model **non-protocol specific** templates and information to sites.
- Develop **informational programs** for investigators and sites staff new to clinical research.

Output

1. **GCP Training Mutual Recognition Program**
2. **8 Site Forms**
3. **12 Informational Programs**
4. **EDC System Training Mutual Recognition Program**

- Today, these solutions are governed by a small team of Member Company Experts. As solutions are integrated with the ***Shared Investigator Platform*** *governance will continue to evolve.*
- Question & Feedback are addressed via SQT@transceleratebiopharmainc.com

Site Qualification & Training | GCP Training Mutual Recognition



What We Did:

- Developed **minimum criteria** based on ICH GCP Guidelines
- Developed a voluntary process for **sponsors to mutually accept** GCP training
- Created an online process for **training providers to self-attest** their training

What we did not do:

- Develop one standardized GCP training course

BENEFITS:

Significant Time Savings: Investigators may only need to re-take GCP training every 3 years and avoid duplicative training.

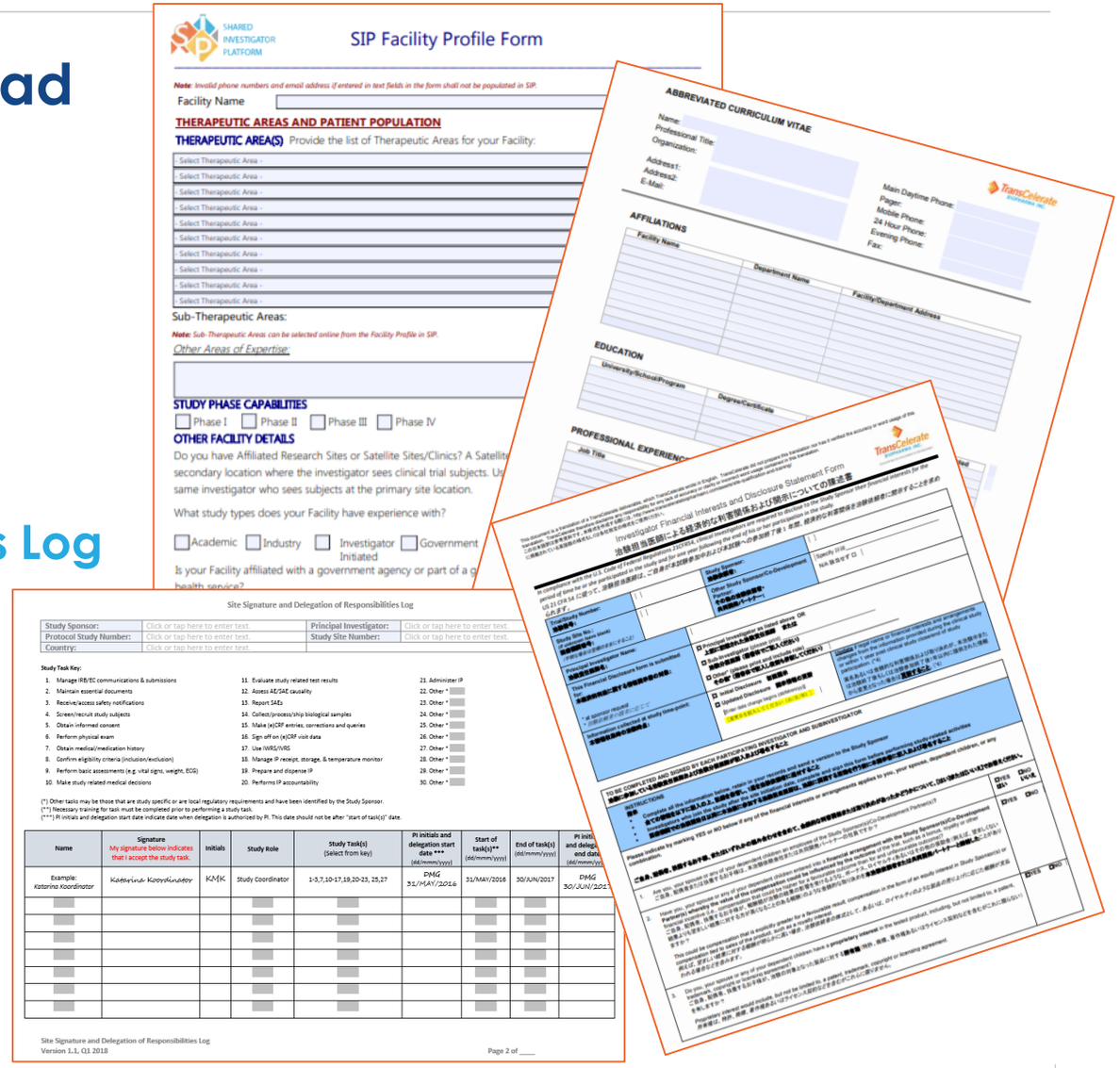
Ease of Participation: Training Providers (e.g. Sponsors, CRO, Hospital, Ethics committee, training consultants, training vendors, etc.) can self- attest via the TransCelerate website.

Site Qualification & Training | Forms for Investigator Sites

Forms and Guidance Available for Download Online:

1. Financial Disclosure Form
2. Curriculum (CV) Template
3. Facility Profile Form
4. Site Signature and Delegation of Responsibilities Log
5. Protocol Level Informed Consent Tracking Log
6. Site Specific Informed Consent Tracking Log
7. Guidance for FDA 1571
8. Guidance for FDA 1572

Select forms have been translated into Japanese



Site Qualification & Training | Informational Program: PI Oversight

What We Did:

- Collaborated with SCRS (Society of Clinical Research Sites) to create an online, **video-based Informational Program** to outline the basic components related to **investigator oversight of clinical trials**
- Provided **examples of tools** that can support investigator oversight
- May be used by investigators that may have **less experience** in conducting clinical trials or for investigators for which oversight has been identified as an issue. May also be used in **preparation for a known inspection or audit**.

All Informational Programs have been translated into Mandarin

BENEFITS:

- **Enhanced patient safety and quality of clinical trial oversight**
- **More effective and efficient study start up and conduct**

Informational Programs are based on ICH GCP E6 (R1) and are under revision to include (R2)

EN NU HET WOORD AAN U…

Voor welke onderwerpen is het van belang dat we die in Nederland meer onder de aandacht brengen van diverse stakeholders? Denk aan de behandelaren, overheid, CRO, Sponsoren, etc.

Patient Technology

Address the barriers to the use of patient-facing technologies (PT) in clinical trials to benefit individual patients and the broader patient community, as well as sponsors, sites, and vendors

Value for Stakeholders

Strives to enable and accelerate use of patient-facing technology in support of an improved patient experience and richer data collection in clinical trials. By fostering industry-wide collaboration regarding the use of patient facing technologies, the initiative intends to:

- Improve efficiency and effectiveness of patient technology implementation by understanding the landscape, sharing implementation experience, and developing tools for multiple involved stakeholders
- Understand and address regulatory uncertainties and barriers
- Develop frameworks for sharing how to use digital means more effectively and efficiently for evidence generation
- Facilitate a patient-centric approach to technology enablement within clinical trials

Patient Experience

Enable greater patient engagement and partnership between patients and sponsors to design and execute clinical protocols that create better patient experiences in clinical trials

Value for Stakeholders

Benefits for patients:

- Increased engagement through better communication and feedback processes
- Increased understanding of the value in participating in clinical trials
- Potential increase in the sense of altruism due to the confidence of knowing that their participation and feedback in trials may improve future study volunteers' experiences
- Potential decrease in the burden of participating in clinical trials

Benefits for sponsors, sites, and investigators:

- Potential improvement in patient recruitment, retention, and adherence within clinical trials
- Potential increase in efficiencies, including possible reduction in long term costs, through more effective patient engagement

eConsent

Create general awareness of eConsent and enable broad, voluntary implementation of eConsent through the development of practical guidance and tools to aid sponsors and other interested parties

Value for Stakeholders

- Provide **patients** clear and easy-to-understand clinical trial information so that they are **truly informed** when making a decision to participate
- Improve patient compliance by offering **sites** the tools to get **insight into patient's understanding** and reduce complex and time-consuming explanations, paperwork and quality risks
- **Reduce** inspection findings and establish submission considerations for **Health Authorities and Ethics Committees** to **improve** review / approval process
- **Enable process efficiencies** (e.g., re-consent, remote monitoring, integration with other eClinical systems), **reduce corrective actions** for audit/inspection consent findings, **improve patient recruitment process** and reduce dropout rates
- **Help aid and support** the transition from paper-based methods to the **use of digital technologies** during the consent process

Protocol Deviations

Work with relevant Health Authorities to create a protocol deviation (PD) management toolkit designed to help sponsors conducting clinical research improve PD classification, collection, analysis and reporting processes

Value for Stakeholders

- Potential **decrease in protocol deviation reporting time**, and improved identification of important protocol deviations
- **Reduction in site burden**, potentially improving a study participant's clinical research experience
- Reduced sponsor burden associated with the interpretation of "important" deviations across all levels of a sponsor organization
- **Focused and efficient management** of protocol deviations which could directly impact patient safety, reliability of study data, human subject protections and / or data quality
- **Decreased internal variability** in protocol deviation definitions and associated site processes for management, reducing confusion, overall burden, and potentially improving a study participant's clinical research experience

Site Qualification & Training

Simplify and enhance the clinical trial qualification and training process by creating efficiencies with a site-level focus

Value for Stakeholders

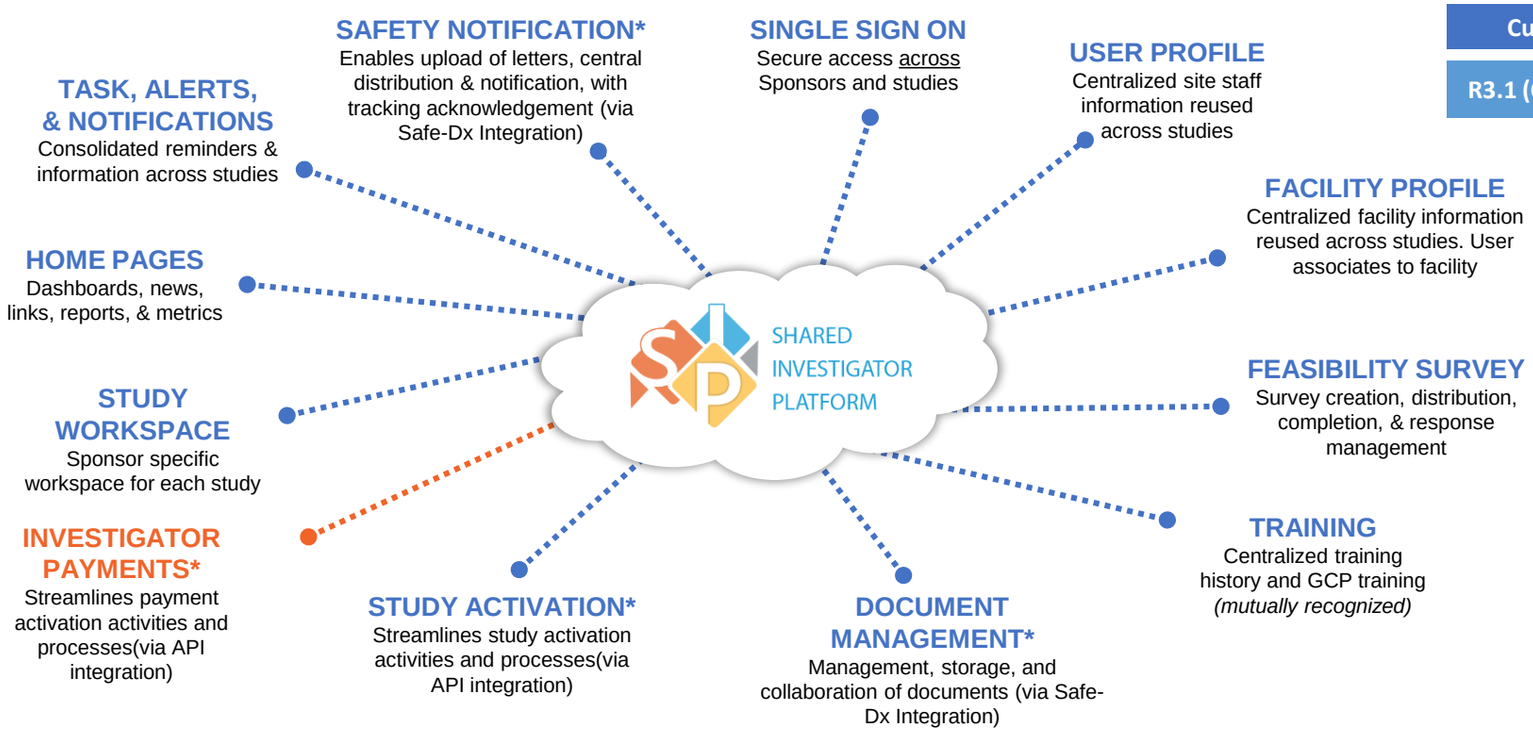
Benefits for sites

- Reduced time spent completing GCP training and redundant training for different sponsors utilizing the same EDC system
- Ease of filling out forms and templates given consistent terminology and approach
- Resources available to support enhanced understanding of the conduct of clinical trials through Informational Programs
- More satisfying clinical trial start-up experience

Benefits for sponsors

- Enhanced study start-up timelines due to ability to mutually recognize GCP training and quickly grant access to EDC systems
- Reduced collection of duplicate information with model forms and templates
- Increased collaboration with sites

Shared Investigator Platform



BULK INVITES
Ability to invite multiple personnel simultaneously to enhance onboarding

SMO/ ARO/ Site Network
Centralized workflows to align with SMOs', AROs', and Site Networks' operating models

ONGOING STUDIES
Enable migration of ongoing studies and relevant information

*Open, Public API

Start enquête……

Q&A