Adaptive master deck – slides are color-coded for specific audiences
✔ = core slides / expected to be included for all audiences
✔ = new audience/unfamiliar w/ TransCelerate & Patient Experience initiative
✔ = content to support the SPFQ Liaison

ACTION REQUIRED:
Red box and fonts from presentation to be removed prior to stakeholder discussions.
The socialization deck offers you introduction materials and links to documents. Use the materials as needed tuned to your audience. It is anticipated that you will not need all of the slides for any one audience. There are several slides that you may wish to customize including the stakeholder template, and unmet needs at your organization.
This deliverable prepared by TransCelerate BioPharma can be adopted by member companies and others, but all adoption is purely voluntary and based solely on the particular company’s unilateral decision. TransCelerate has provided this Study Participant Feedback Questionnaire (“SPFQ”) and the corresponding User Guide (collectively the “Work Product”) for informational purposes only. By using the Work Product, you manifest your assent to the terms of use set out in this paragraph. The Work Product are not tailored to any particular factual situation and are provided ‘AS IS’ WITHOUT WARRANTY OF ANY KIND, EITHER EXPRESSED OR IMPLIED, INCLUDING, BUT NOT LIMITED TO, THE IMPLIED WARRANTIES OF FITNESS FOR A PARTICULAR PURPOSE, NONINFRINGEMENT, OR MERCHANTABILITY. TransCelerate and its members do not accept any responsibility for any loss of any kind including loss of revenue, business, anticipated savings or profits, loss of goodwill or data, or for any indirect or consequential loss whatsoever to any person using the Work Product. Any party using the Work Product bears sole and complete responsibility for ensuring that the Work Product, whether modified or not, are suitable for the particular clinical study, accurate, current, commercially reasonable under the circumstances, and comply with all applicable laws and regulations.
AGENDA/CONTENTS

- Introduction to TransCelerate & Patient Experience Initiative Overview
- SPFQ Toolkit Elevator Pitch
- SPFQ Toolkit Overview
- SPFQ Toolkit Value Proposition
- SPFQ Toolkit Liaison Support
Introduction To
TransCelerate
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 The Organization

2012
TransCelerate Founded

2016
BioCelerate Founded

Today

20 MEMBER COMPANIES

25+ INITIATIVES

MEMBER COMPANIES

Regeneron most recent member

INITIATIVES

including 4 pharmacovigilance initiatives

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

TransCelerate

focus on preclinical research

BioCelerate

BioCelerate Founded

Focus on preclinical research

As of May 2019
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*.

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

As of May 2019
Introduction To The Patient Experience (PE) Initiative
Why Did the Industry Need A Patient Experience Initiative?

Patients understand and expect more from drug development

- Technology advances & access to information about diseases & drug development
- Biomarker-driven personalized medicine development

Based on responses from 14 companies, sponsors don’t have regular processes for engaging patients in study design & conduct. Of the 14 sponsors surveyed:

- 1 of 14 sponsors surveyed received feedback from patients who declined to participate in a clinical study
- 10 of 14 sponsors surveyed didn’t ask for feedback about logistics during clinical studies
- 0 of 14 sponsors surveyed had a consistent, company-wide process for asking feedback from clinical study participants
- 3 of 14 sponsors surveyed received feedback after a clinical study ends

Dietrich et al., 2017, Therapeutic Innovation & Regulatory Science 51(5)
TransCelerate’s Patient Experience Near And Longer-Term Goals

TransCelerate’s Patient Experience Initiative seeks to develop tools to provide more effective ways to engage with patients in the design and execution of clinical studies.

By designing clinical studies with patient inputs, study sponsors can potentially...

- Improve the patient's experience in clinical studies
- Increase the number of patients willing to participate in clinical studies
- Reduce protocol amendments and study participant dropout rates
- Speed up the delivery of medicines to market
- Support development of more ‘fit for patient’ drugs

Near-Term Goals

Long-Term Goals
Introduction to Patient Experience (PE) Initiative

To improve engagement and partnership between biopharmaceutical companies and patients to create better experiences for clinical study participants

VISION

Develop better understanding of how...

Sponsors, Sites, Investigators

Diverse Patient Populations

Translates into...

Reduced Patient Burden
Perception of Value
Patient Empowerment
Willingness to Participate
Patient Inputs Will Allow Sponsors to Improve the Patient Experience in Clinical Studies

Potential **decrease in burden** of participating in clinical studies

Potential to positively impact **patient adherence and compliance** to clinical study procedures

Increased **trust and engagement** through better communication and participation in feedback processes

The cycle of engaging patients and evaluating their clinical study participation feedback can allow clinical protocols to become more **patient-centered**

Potential **increase in the sense of altruism** due to the confidence of knowing that their participation in studies may improve future study participants’ experiences
The primary goal of patient-focused drug development is to better incorporate the patient’s voice in drug development and evaluation, including but not limited to:

- Facilitating and advancing use of systematic approaches to collecting and utilizing robust and meaningful patient and caregiver input to more consistently inform drug development and regulatory decision making
- Encouraging identification and use of approaches and best practices to facilitate patient enrollment and minimizing the burden of patient participation in clinical studies
- Enhancing understanding and appropriate use of methods to capture information on patient preferences and the potential acceptability of tradeoffs between benefit and risk outcomes of the study intervention
- Identifying the information that is most important to patients related to treatment benefits, risks, and burden, and how to best communicate the information to support their decision making.

The patient engagement tools will contribute to an improved partnership between sponsors and patients in clinical studies.

**Patient Protocol Engagement Toolkit (P-PET)**
A clinical study engagement toolkit composed of:
- Sponsor-facing operational user guide
- Resource guide with question bank
- Templates to enable study sponsors to engage with patients during clinical study design.

**Study Participant Feedback Questionnaire (SPFQ) Toolkit**
A clinical study participant feedback toolkit composed of:
- Socialization deck for initial Sponsor discussions
- Sponsor-facing operational user guide
- Set of 3 Study Participant Feedback Questionnaires (beginning, during and end of study)

Design clinical studies with patient inputs

Gather patient feedback during clinical studies
Time-Point Considerations For Implementing Patient Experience Tools In Clinical Studies

Patient Protocol Engagement Toolkit

- P-PET
  - Target Product Profile
  - Clinical Development Plan
  - Protocol Concept
  - Protocol Optimization

Design clinical studies with patient input

Study Participant Feedback Questionnaire Toolkit

- SPFQ
  - Protocol Concept
  - Protocol Execution
  - Data Analysis
  - Data Dissemination
  - Post Study

Gather patient feedback during clinical studies

Design clinical studies with patient input

Gather patient feedback during clinical studies
SPFQ – Elevator Pitch
SPFQ Elevator Pitch

The SPFQ is a questionnaire given to patients at the beginning, during and end of a clinical study so sponsors can improve studies by learning from patients.
SPFQ Toolkit – Overview
SPFQ Toolkit: Objective & Overview

- The TransCelerate Patient Experience Initiative developed a Study Participant Feedback Questionnaire (SPFQ) Toolkit designed to:
  - Facilitate sponsors to collect real-time feedback from clinical study patients
  - Identify burden/impact to inform steps to make studies less burdensome to patients

- The SPFQ Toolkit has been developed with patients:
  - Inspired by patients
  - Developed with patient input throughout the process

- The SPFQ Toolkit has the potential to help a sponsor:
  - Assess the patient’s experience in a study
  - Enable real-time feedback from study participants
  - Identify steps to make future studies less burdensome to patients
  - Leverage aggregated SPFQ data across a given sponsor’s studies to inform actions to improve patient experience in a clinical development program

- The SPFQ Toolkit contains:
  - Questionnaires (study start-up, during, end of study)
  - Sponsors’ User Guide to support implementation of the SPFQ in a study
  - Socialization deck
Developing The SPFQ Toolkit

Develop 1st Draft SPFQ Toolkit
- SPFQ items v0.1
- User Guide v0.1
- Explore data collection options

Q1-Q3 2018
- Test & Refine SPFQ v0.1 items
  - Quantitative item testing
  - Cultural & geographic confirmation
  - SPFQ v0.1 items
  - Engagement with SAGs and CROs as appropriate
- Internal Functional Stakeholder Interviews Round 1
  - Refine & modify the SPFQ Toolkit User Guide
  - Identify “other” support materials needed

Q4 2018 – Q2 2019
- Internal Functional Stakeholder Interviews Round 2
  - Review updated user guide after Round 1 input
  - Feedback for additional updates
  - Identify study team for initial use

SPFQ Toolkit
- SPFQ
- Socialization Deck
- User Guide
- Piloted toolkits with member companies
- Design study-specific implementation process

Global Relevancy
- Update the SPFQ v1.0 by ensuring content and cross-cultural validity through concept confirmation interview of a diverse population (non-English speaking)

As of May 2019
Sample SPFQ Items
Refer to the questionnaire for the complete set of items

<table>
<thead>
<tr>
<th>Start of study</th>
<th>Strongly disagree</th>
<th>Disagree</th>
<th>Neither agree or disagree</th>
<th>Agree</th>
<th>Strongly Agree</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>A1.</strong> I understand the treatment process in this trial (for example: when and how to take or use a treatment)</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>During study</th>
<th>Strongly disagree</th>
<th>Disagree</th>
<th>Neither agree or disagree</th>
<th>Agree</th>
<th>Strongly Agree</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>B1.</strong> Overall I am satisfied with the trial site (for example: comfort and privacy of treatment area, waiting area, parking, ease of access to the site)</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
</tr>
<tr>
<td><strong>B2.</strong> My trial visits have been well organized</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>End of study</th>
<th>No</th>
<th>Yes</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>C1.</strong> I was informed when I had completed the trial</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>C2.</strong> I was informed of any future opportunities to access the overall trial results if I wanted to</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
SPFQ – Value Proposition
Problem Statement

The experience of patients participating in clinical studies may not consistently meet expectations and can be inconsistent with a culture of patient-centricity. Unsatisfactory patient experience is a material negative influencer of recruitment, retention, and reputation.

Solutions

- An easy way to measure participant satisfaction as part of a comprehensive strategy of patient engagement to design patient centric studies and to inform current and future studies.
- Identify patient satisfaction measures and barriers to participation in clinical studies across therapeutic areas and sponsor companies regarding their experience in clinical studies and potentially improve sponsor reputation.
- Potential mitigation of patient dissatisfaction outcomes such as study drop out, decreased adherence, common retention challenges.
- Provide insight into the actual needs of the participant and offer an opportunity for sponsors to respond with patient centric solutions that meet patients’ lifestyle and medical needs.
Study Participant Feedback Questionnaire vs. Alternates

The TransCelerate Patient Experience team is aware that there are other initiatives and questionnaire offerings within industry use or development. While we support and learn from this landscape, the SPFQ Toolkit should be differentiated through:

- Cross-functional, consortia development of the user guide to streamline and ease adoption that may be adapted to each sponsor’s needs
- Flexibility on how sponsors can administer through their platform of choice
- Validated questionnaire with publication history that the TransCelerate team is further developing through global concept confirmation
- Potential to have additional translations which are shared back to TransCelerate by sponsors or other stakeholders
Filling A Patient Experience Unmet Need At [Sponsor]

- Consider describing the Patient Experience landscape within your organization
- Consider if or how study experience is being evaluated by your organization
- Consider how the SPFQ Toolkit fulfills any gaps for your organization
Implementation Considerations

3 major questions:

1. What are the key initiative sub steps required for success?
   Questions to consider:
   • What are the key bodies of work?
   • What is the sequence of work?
   • What work has already been done?

2. What are the key initiative sub steps required for success?
   Questions to Consider:
   • What type of resource do you need?
   • How many of each type of resource do you need?
   • What can it be done internally? What requires contractors?
   • How much time will it take?
   • What vendors are required? For how long?
   • What hardware or software is required?

3. What are the implications for outside resources for implementation?
   Questions to consider:
   • Will there be a need for contractors?
   • Over what time will external vendors be needed?
   • What is the infrastructure need?
Use of the SPFQ in clinical studies and programs will contribute to patient-centric clinical studies potentially leading to:

- Improved patient experience
- Enhanced patient recruitment into clinical studies
- Improved adherence in clinical studies
- Reduced patient drop-out
- Enhanced data quality
- Reduced number of amendments
- Development of drugs valuable for patients may result in
  - Reduced cycle times
  - Reduced overall clinical study times
  - Contribution to improved patients’ adherence to medication
SPFQ Toolkit – Liaison Support
How Do I Use The SPFQ Toolkit?

This toolkit is intended to support you as a SPFQ liaison to facilitate the internal socialization of the SPFQ to your colleagues, and assist in the start-up of the questionnaire execution. The toolkit includes the following:

1. The socialization deck offers you introduction materials to share with your organization. Use the materials as needed for your target audience. It is anticipated that you will not need all of the slides for any one audience. There are several slides that you may wish or need to customize including the stakeholder template, and unmet sponsor needs.

2. The user guide is the primary tool for study teams that are considering the implementation of the SPFQ.

3. Set of 3 Study Participant Feedback Questionnaires (SPFQ) to administer at the beginning, during and end of a study.
Organization Liaison Considerations

Adoption of SPFQ Toolkit
- Raise awareness to Key Stakeholders/Leadership
- Identify your executive management champion to support implementation at your organization
- Consider developing a core team including organization champion, data services, study management and legal/regulatory that you meet with periodically

Roll-out
- Raise awareness to organization target adopters
- Identify study teams to implement
- Organization liaison provides training

Support
- Hold routine meetings with study teams
- Provide 1:1 support with Q&A
- Share lessons learned and best practices within your organization

Cross Functional Stakeholder Template

<table>
<thead>
<tr>
<th>Core Team</th>
<th>Compliance Stakeholders</th>
<th>Advisors</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Project Manager</td>
<td>• Legal</td>
<td>• Internal Champion</td>
</tr>
<tr>
<td>• HEDR / COA</td>
<td>• Privacy</td>
<td>• Finance</td>
</tr>
<tr>
<td>• Trial Management</td>
<td>• Safety</td>
<td>• Procurement</td>
</tr>
<tr>
<td>• Trial Monitoring</td>
<td></td>
<td>• Patient Relations</td>
</tr>
<tr>
<td>• Data Management</td>
<td></td>
<td>• Patient Engagement</td>
</tr>
<tr>
<td>• Clinical Development</td>
<td></td>
<td>• Innovation</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• IT</td>
</tr>
</tbody>
</table>
For more information about TransCelerate, visit us: www.TransCelerateBioPharmaInc.com

Watch our “About Us” Video

Sign up for our Newsletter, Accelerate to Innovate