Patient Protocol Engagement Toolkit (P-PET) User Guide

Version 1
Legal Disclaimer

The deliverables of the TransCelerate initiatives 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 BioPharma has provided this Patient Protocol Engagement Tool 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 NON-INFRINGEMENT, 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 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.
Table Of Contents

Introduction to TransCelerate & the Patient Experience (PE) Initiative

Introduction to the Patient Protocol Engagement Toolkit (P-PET)

P-PET User Guide

Use Cases

Resources and References
Introduction To TransCelerate
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
10
MEMBER COMPANIES
5
INITIAL INITIATIVES

2016
BioCelerate Founded

2016
BioCelerate
focus on preclinical research

Today

20
MEMBER COMPANIES

25+
INITIATIVES

Regeneron most recent member
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

Copyright ©2018 TransCelerate BioPharma Inc., All rights reserved.
Confidential

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*.

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.
**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*

**ENHANCE SPONSOR EFFICIENCIES & DRUG SAFETY**
- Advancing Safety Analytics
- Clinical Data Transparency
- Data Monitoring Committee
- Intelligent Automation Opportunities in Pharmacovigilance

**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

*New Work approved by TransCelerate Board for 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 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...

- Study Design
- Scientific Objectives
- Procedures & Assessments
- Schedule of Activities

Translates into...

- Reduced Patient Burden
- Perception of Value
- Patient Empowerment
- Willingness to Participate

Sponsors, Sites, Investigators

Diverse Patient Populations
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
Patient Experience Initiative Goals Align with PDUFA VI/21st CCA Commitments

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.

Green text relevant to Patient Experience Initiative objectives
Source: https://www.fda.gov/Drugs/DevelopmentApprovalProcess/ucm579400.htm
Patient Experience Initiative

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)
Time-Point Considerations For Implementing Patient Experience Tools In Clinical Studies

Patient Protocol Engagement Toolkit

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

Design clinical studies with patient input

Study Participant Feedback Questionnaire Toolkit

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

Gather patient feedback during clinical studies
Introduction To The Patient Protocol Engagement Toolkit (P-PET)
TransCelerate has developed a toolkit to embed patient perspective into clinical study protocols and the drug development process.

The patient perspective on patient experience and burden should be central to the drug development process.

The TransCelerate Patient Protocol Engagement Toolkit (P-PET) makes available tools and resources to use in engaging patients during protocol development with the goal to improve patient experience and reduce patient burden as a study participant.

The P-PET can be utilized by sponsors to partner with patients in creating patient-centric clinical studies.
Introduction to the Patient Protocol Engagement Toolkit (P-PET)

The Patient Protocol Engagement Toolkit (P-PET) is a comprehensive set of materials that can help biopharmaceutical companies and other Stakeholders engage with patients during the study design phase (e.g., as early as protocol concept).

Components of the P-PET include:

- **User Guide** - The User Guide supports sponsors in (1) understanding the value of implementing the P-PET in their respective clinical studies, (2) understanding how to leverage the P-PET and implement it in a clinical study and share ideas and best practices on how to have meaningful discussions with patients, (3) socializing with stakeholders to seek greater support as needed, and (4) providing example case studies.

- **Resource Guide** - A set of sample questions for consideration during engagement with the patients. Examples of visual aids are provided to facilitate clear communication of study design and protocol-related concepts to the patients.

- **Templates** - Templates (Patient Advisory Board (PAB) Pre-read, PAB Presentation, PAB Satisfaction Survey: Patient, PAB Satisfaction Survey: Study Team, PAB Thank You Note, PAB Patient Report, PAB Study Team Report) to help support engagement discussions with patients and provide feedback to both the study teams and patients.

Sponsor teams responsible for the design, planning, and conduct of a clinical study/clinical program should consider implementing patient engagement as early as possible in the protocol development lifecycle. The sponsor team could also conduct an assessment of internal support and available resources, including necessary budget, to support this engagement process. This includes identifying the key roles and responsibilities that drive the engagement process with patients.
An Example Of How The P-PET Might Work:

This is **Jan**. She is a **researcher** helping to design a clinical study for a **biopharmaceutical company**.

This is **Amy**. She is a **patient** who has experienced the condition being researched.

**Jan** in collaboration with a patient engagement **moderator/facilitator** will use the **P-PET** to get **feedback** about the **protocol** from **Amy**.

**Jan** will use the feedback to help improve the **design and conduct** of the study.
### Start-Up Considerations

<table>
<thead>
<tr>
<th>Consider leadership buy-in</th>
<th>Consider your internal SOPs and legal guidance as well as other stakeholder approvals as required</th>
<th>Consider budgetary needs, timelines, and resource allocation, including need for 3rd party vendor support</th>
<th>Consider IRB/Ethics Committee review</th>
</tr>
</thead>
<tbody>
<tr>
<td>Consider disease knowledge and patient population insights that may already exist within your sponsor company and how this toolkit may help complement existing insights</td>
<td>Consider how the data resulting from patient engagement activities will be collected, shared, stored, assessed, and utilized to design a patient-centric study</td>
<td>Consider who will be the owner of patient experience and engagement activities</td>
<td>Consider establishing roles and responsibilities that will support the process</td>
</tr>
</tbody>
</table>
Note for Sponsors

While reviewing the P-PET User Guide please remember that:

• The use of “patient” throughout can refer to anyone speaking for or representing a patient, for example: patients, caregivers, parents, patient advocates, patient advisors, or expert patients.

• A Patient Advisory Board may include “patients” who are living with the condition and/or participating in the specific study and/or patients who are eligible for the study.

• We are asking the “patient” to imagine that they are planning to take part in the study.
P-PET User Guide Introduction

The User Guide provides considerations on why, when, and how to engage with patients during study design to enhance the quality and efficiency of clinical development activities and results.

As a component of the P-PET, the User Guide provides a step-by-step process flow with recommendations on how to effectively conduct patient engagement activities.

This User Guide shares ideas around how to have engaging and beneficial discussions with patients.

There are several case studies for your sponsor reference that will help to illustrate various types of patient engagements.
Include Patient Insights Early In Program Development And/Or Study Planning

- If there is an internal process or contracts established, an online survey potentially could be conducted in ~12 weeks; virtual meetings ~8 weeks (includes preparation time with the study team).
- Start Planning as Early as Possible*, Iterative Process; More than One Interaction with Patients Possible
- Select the Best Patient Engagement Method to Achieve the Goals
- Conduct Patient Insight Work with Selected Approach
- Review Patient Insight Output
- Apply Insights

*Planning for patient input into Phase 3 Protocols should begin toward the end of Phase 2

- Review results from patient insights to agree on protocol modifications and any additional support to be provided during study conduct allow 2-4 weeks to discuss depending on extent of protocol comments.
- If no internal process or contracts established to conduct insight method planned, may need 9-12 months prior to final protocol to gather patient insights.
Process Flow For Patient Engagement Activity

1. Identify Need & Desired Goals (What to consider)
2. Select Patient(s) & Appropriate Engagement Method
3. Set Up Patient Engagement
4. Engage Patient(s) *
5. Assess Feedback*, Follow-up, & Apply Learnings *

*This is an iterative process.
Considerations For Engaging Patients

Define your objective and scope

- **Consider:**
  - Do you have previous experience in this patient population/indication?
  - What is your patient population & where are they (e.g., geography, specialty, any cultural issues to address)?
  - What are the objectives of your patient engagement activity?
  - What is the best engagement method?

- **What are the specific questions you want answered?**
  - (e.g., understanding of patient journey, assumptions vs. real life, medical practice pattern)
  - Do you have key design elements that may negatively impact the patient (schedule of assessments)?

- **Consider any study-specific needs**
  - (e.g., ePRO, draft ICF, drug packaging, use of devices)

- **Define roles and responsibilities**
  - Consider appointing a Patient Engagement Lead who will manage the patient insights work, and engage with the internal team and relevant stakeholders.
Things To Consider When Selecting Patients

- Having patients be part of your clinical study planning can provide important insights. Carefully selecting patient partners is a critical step to obtaining meaningful patient insights.

Consider the following patient criteria when evaluating the types of patients to best support your needs:

- Your access to patients (via patient groups, patient advocates) or need to engage with 3rd party vendor
- Need to engage with caregivers in addition to/instead of patients
- Where patients are in their patient journey (influencers, family dynamics, community)
- Variety of treatment experience
- Disease state/co-morbidities
- Diversity (demographics, education, literacy levels, socio-economic backgrounds, geography, language)
- Perception of clinical studies specific to patient population
- A mix of naïve and experienced patients/advocates to create a comprehensive voice
- Patients with varying degrees of exposure to/involvement in clinical studies
What Type Of Patient Engagement Method Will Help Achieve The Objective(s)?

- Consider the internal budget and timelines

- Consider the outcome you hope to gain to help determine meeting type
  - Direct vs. indirect patient engagement:
    ✓ Will 3rd party moderator be utilized or will sponsor company facilitate and lead?
    ✓ Sponsor name may be preferred to be kept anonymous to patients as to not create bias
    ✓ Ensure all present are well prepared to have effective interactions with patients (e.g., with appropriate language and reactions).
  - Regardless of the type of meeting, be sure to prepare engaging interactive activities to get patients comfortable discussing themselves and their condition
  - Refer to the Methods of Engagement table on [next slide](#)

- Consider having an experienced moderator/facilitator, as this is highly recommended for all meetings with patients. Moderator/facilitator should be able to create a comfortable environment and should not use corporate jargon/acronyms.

- Consider scheduling engagements so relevant sponsor team members can engage firsthand or observe the interactions with patients (e.g., regulatory, clinical development leads, operational lead, etc.)
### Example Methods Of Engagement

<table>
<thead>
<tr>
<th>Example Engagement Types</th>
<th>Description</th>
<th>Resource Burden</th>
<th>Cost</th>
<th>Time</th>
<th>Proposed Use</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient Advisory Board (PAB)</td>
<td>Live meeting. Multiple patients and sponsor representatives</td>
<td>High</td>
<td>High</td>
<td>High</td>
<td>Use with new indication (no prior sponsor knowledge) and/or high number of consideration needs identified. Patient Advisory Boards typically remain available for a contracted period of time as protocol consultants.</td>
</tr>
<tr>
<td>Focus Group/ Interviews</td>
<td>Live meeting. Multiple patients and sponsor representatives</td>
<td>High</td>
<td>Med</td>
<td>High</td>
<td>Use with new indication (no prior sponsor knowledge) and/or high number of consideration needs identified.</td>
</tr>
<tr>
<td>1:1 Face-to-Face Meeting</td>
<td>Live meeting. Single patient and sponsor representative(s)</td>
<td>Med</td>
<td>Med</td>
<td>Med</td>
<td>Use when it is more practical for interviewer to travel to patients (e.g. some diseases may limit ability of patients to travel). Use when questions to be answered include being able to observe a patient in their environment.</td>
</tr>
<tr>
<td>Virtual Meeting (e.g., WebEx)</td>
<td>Engaging with patients via the internet using integrated audio and video</td>
<td>Med</td>
<td>Med/Low</td>
<td>Med</td>
<td>Use when travel may be difficult for patients or there are budget and/or time limitations. Allows for more flexibility to hold the WebEx outside of working hours to be respectful of the patient’s time.</td>
</tr>
<tr>
<td>Social Listening/ Social Media</td>
<td>Utilizing social media to gain patient insights</td>
<td>Med</td>
<td>Med</td>
<td>Low</td>
<td>Use when a quick read on patient perspective is needed. What are patients talking about, what are some key/current concerns, etc.?</td>
</tr>
<tr>
<td>Questionnaires/ Surveys</td>
<td>Engaging with patients in a live meeting or virtually via a series of questions/ratings to gain patient feedback</td>
<td>Low</td>
<td>Med</td>
<td>Low</td>
<td>Use when feedback is desired from large number of patients. Questions can be answered with multiple choice, rank order, minimal open text fields (the latter difficult to analyze).</td>
</tr>
</tbody>
</table>
Considerations for conducting in-person patient engagement

Legal considerations:

✓ HIPAA compliance considerations – photo releases/policy, use of first names only.....
✓ Data privacy law considerations

Flow of the meeting:

✓ Proposed agenda items: Welcome, Introductions, Overview/Purpose of Engagement, Meeting Format & Ground Rules.
✓ Provide a clear purpose for the engagement.
✓ Allow room in the agenda for general discussion time with patients. If time allotted during the engagement does not allow for this, consider a pre-engagement interaction to allow patients to discuss general topics in advance.
✓ Consider methods that will enhance open discussion, such as ice-breaker exercise, dropping titles and using first names, giving the patients the opportunity to share their personal disease journey.
Other engagement considerations (method of engagement dependent)

- **Number of participants** - ratio of patients vs. sponsor staff
- Ensure a **patient-friendly session**: The patient engagement planning stage is your first opportunity to engage with patients - to be patient-centric. **Ask them what they need!**
- Patients are people: Identify if there are **specific participant needs** that should be accommodated during the engagement. For example:
  - For patients with IBD, ensure that restrooms are located near the meeting room.
  - For patients with lupus, start the meeting later in the day and allow several breaks.
  - Understand if they have any dietary restrictions, ambulatory restrictions, etc.
- **Atmosphere should be comfortable and casual.**
- Engagement **sessions** should be appropriate in **length with frequent breaks**
  - When creating agenda, consider the patients’ need to “share their story” and experience.
  - Ensure sufficient time is built into the agenda to have appropriate interactions (every interaction is an opportunity to learn from the patients).
- Consider providing **pre-read materials** to the patient engagement participants.
  - Agenda, Informed Consent document, example of questions that will be asked, example of patient recruitment materials.
Patient Engagement Logistics (3 of 3)

- Patient Engagement Lead / Project Manager: someone to drive the planning and execution of the patient engagement activity
- Subject Matter Expert in Patient Engagement: sponsor company advisor
- Study team member(s): ensure the ratio of patients vs. team members is appropriate
- Patients (e.g., via a Patient Advocacy Organization)
- An appropriate Moderator/ Facilitator/ Interviewer
  - Find an experienced moderator who knows how to engage participants and can bring out relevant patient experience, can explain concepts and goals in digestible terms, and can facilitate patient engagement in a controlled, meaningful, and balanced manner
- A Meeting Scribe for taking notes/recording feedback during engagement for collating patient feedback/raw data
- Translator and/or Interpreter
- Other roles, as applicable
Considerations For Framing Your Objectives

- Reaction to the study design elements after being provided with thorough information, including the patients’ perceived benefit-risk of participation in the study.
- Gain insight into the disease burden and help understand the impact of the illness on the patient, the family, and the caregiver.
- Plans, hopes, and concerns related to their disease/condition.
- Identify potential obstacles and/or hardships pertaining to study-related tests, procedures, and/or visits.
- Opportunities to review and provide feedback on clinical-study-related documents

*What kind of insights are you looking for from patients?*

*The P-PET Resource Guide is a helpful reference when answering this question.*
Refining The Questions To Ask

Understand their knowledge of clinical studies, country specific needs to consider and willingness to consider a clinical study for their disease/condition.

How would they assess whether to participate or not?

What are the barriers and motivators for participation? How do patients learn about the clinical trial?

Ask the participants if there is anything important to them that hasn’t been considered!

Refer to the P-PET Resource Guide & Question Bank; select relevant questions and modify/customize as needed.
## Considerations During Engagement With Patients

<p>| | | | |</p>
<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>1</strong></td>
<td><strong>2</strong></td>
<td><strong>3</strong></td>
<td><strong>4</strong></td>
</tr>
<tr>
<td>Set expectations with patients about the engagement process: what happens, when it happens</td>
<td>Explain <em>where their input will go</em> and how it remains confidential</td>
<td>Acknowledge that each person’s situation and input is <em>unique and valued</em></td>
<td>Encourage an atmosphere of openness - remove judgment and disagreement to avoid shutting down any patients with divergent experiences</td>
</tr>
<tr>
<td><strong>5</strong></td>
<td><strong>6</strong></td>
<td><strong>7</strong></td>
<td></td>
</tr>
<tr>
<td>Encourage those with differing opinions and experiences to share alternate viewpoints</td>
<td>Consensus is not the goal – <em>walking in their shoes</em> should be</td>
<td>Be aware of patients who dominate conversation or are reticent to voice their contributions to ensure all patient voices are heard</td>
<td></td>
</tr>
</tbody>
</table>

*Consensus is not the goal – *walking in their shoes* should be*

*Be aware of patients who dominate conversation or are reticent to voice their contributions to ensure all patient voices are heard*
Outcomes/Next Steps: Sponsor Stakeholders

Scribe provides engagement output to sponsor team (compile raw data)

Review output with sponsor engagement team
- Ideally within first 2 weeks post-engagement
- Organize the engagement output for team use (Final Report) and include insights and interpretations
- Determine suggested actions
- Consider regulatory impact

Disseminate final report to key sponsor stakeholders
- Ideally within 2 weeks post-sponsor engagement team review
- Evaluate suggested design changes and potential downstream impact
- Apply and document agreed-upon changes
- Track the impact of the patient engagement on key study performance indicators
Outcomes/Next Steps: Patient

Consider administering a Satisfaction Survey
- Timing dependent on engagement type
- Survey can be for the patient and study team

Show appreciation to the Patients for the support
- Ideally within 24-48 hours post engagement

Communicate back to the Patients
- Summarize where their feedback was utilized and/or how it influenced protocol design or other study elements (where appropriate).
- Consider to include where feedback could not be used, as appropriate, and the rationale.
- Consider continuing to communicate with the patients throughout the lifecycle of the study, including awareness regarding why the study was discontinued early (as applicable).
Applied Learnings: Value Realization

- Logistical lessons learned / challenges
- Future process improvements
- Distribute lessons learned to appropriate sponsor stakeholders
- Best practice refinement
- Suggested changes from patients that have broader reaching impact to the sponsor

Utilize any documented changes for potential future regulatory and ethics interactions
Use Cases
Background

In these use case slides* you will find:

• Examples of particular problems encountered by sponsors
• Example insights gathered by the sponsors as a result of patient engagement
• Example actions taken by the sponsors as a result of the patient engagement insight

*currently these use cases are from methods prior to P-PET development
Use Case-Procedures

Timing/Volume of Procedures Used in a Clinical Study

Problem:

• Study team was unsure of both the number and timing of procedures
• Were they out of sync with the practical realities of the health care system or patient lives?

Insight:

• Patients provided their comments on the acceptability of the proposed procedures and associated timings

Action:

• Through patient and site collaboration, the sponsor redesigned the schedule of procedures.
Invasive Procedures

Problem:

• Study team was considering requiring an invasive procedure in immunocompromised patients

Insight:

• Patients informed sponsor that this would negatively impact willingness to participate

Action:

• Protocol was changed to make the procedure optional
Use Case-Drug Appearance

Drug Appearance

Problem:
• Multiple pills involved in treatment therapy were similar in appearance
• Presented a new opportunity for drug manufacturing to explore how they might consider patient input in their work beyond size and taste of a pill

Insight:
• Patients helped to create solutions to better discern the different pills and any associated activities

Action:
• Creative solutions were implemented to help improve drug compliance and prevent possible medication error
Use Case—Reducing Patient Burden

Reducing Patient Burden

Problem:
• Patients’ perceive a high burden in participating in a clinical trial

Insight:
• Proposed duration of visits and frequency of tests were burdensome
• Patients appreciated proposed ePRO

Action:
• Reduced duration of visits and frequency of tests, as time burden was a key patient concern
• Decision to proceed with ePRO in response to positive patient feedback

Outcome:
• Reducing burden and improving the patient experience led to reduced screen failures, reduced drop-outs, increased enrollment rate, 0 avoidable amendments
Use Case - Patient Reported Outcomes

Patient Reported Outcomes (PROs)

Problem:
- Study team wanted to further understand what type of outcomes from their treatment mattered most to patients, including physical, emotional, and social functioning

Insight:
- Interviews & surveys (with patients and clinicians) highlighted the importance of the ability to function and partake in daily activities

Action:
- Developed Diary instrument to capture these PROs during trials
- Developed Recorder App as a clinical practice tool to capture patient experience

Outcome:
- Included objective data on the results for the endpoint in the label
Use Case-Diverse Populations

Diverse Populations

Problem:
• Study team wanted to determine how to meet the needs of a diverse patient population

Insight:
• Gained information on patients' overall perspective of clinical studies, their decision making process, barriers, and motivating factors impacting participation and raising disease awareness

Action:
• Focused efforts on educational initiatives related to patients' general knowledge and perceptions of clinical trials and educated investigators on patient educational needs while enrolling
• Worked to facilitate the use of more diverse imagery in clinical trial materials
• Added as much specific information related to prior trials and the present trial to patient materials as possible
• Included patient feedback during development of patient brochures and other educational materials
Use Case - Exit Interviews

Exit Interviews

Problem:

• Study team wanted to understand the patient perspective on the clinical trial experience

Insight:

• Important concepts and dominant trends in each interview were identified, and results were compared across interviews to allow for the generation of themes or patterns in participants’ responses

Outcome:

• Qualitative information provided additional data to assist in interpreting the quantitative data from the trial, giving in-depth understanding of patient experiences, and can be used for future hypothesis generation
Resources and References
Patient Advisory Boards (PABs) are a valuable way of implementing patient insights into the drug development and clinical study processes. The constitution of your PAB should reflect your study population in general. Below are several links to whitepapers or resource materials for a PAB


Patient Centered Primary Care Collaborative on starting/sustaining PABs: https://www.pcpcc.org/resource/starting-and-sustaining-patient-advisory-board

FDA Patient Engagement Advisory Committee Meeting Materials: https://www.fda.gov/AdvisoryCommittees/CommitteesMeetingMaterials/PatientEngagementAdvisoryCommittee/ucm578522.htm

Engaging Patients As Partners (Resources For Sponsors)


Patient-Centered Outcomes Research Institute (PCORI) has a searchable list on patient engagement publications: https://www.pcori.org/literature/engagement-literature

Engaging Patients As Partners (Resources For Sponsors)

EUPATI  https://www.eupati.eu/


IMI Paradigm  http://imi-paradigm.eu/

Patient Focused Drug Development (PFDD)
https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

Patient Focused Medicines Development
https://involvement-mapping.patientfocusedmedicine.org/initiatives
For a patient to be successful contributing to the development of therapeutics, it may be helpful to provide some background knowledge of the development life cycle. Providing patients with a pre-read to acquire basic understanding of the drug development process may help the patients acclimate.

FDA.gov Drug Development Process (a good, high-level overview of the development process and regulatory approval, in simplistic terms): https://www.fda.gov/ForPatients/Approvals/Drugs/default.htm


Patient Advisor Role (Henry Ford Health Care) video: https://www.youtube.com/watch?v=vWxWcMhvsyk

For more information about TransCelerate, visit us:
www.TransCelerateBioPharmaInc.com

Watch our “About Us” Video

Sign up for our Newsletter,
Accelerate to Innovate