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 Toolkit and this corresponding Resource 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.
The vision for TransCelerate’s Patient Experience initiative is to improve engagement and partnership between biopharmaceutical companies and patients to create better experiences for clinical study participants.

To achieve this, TransCelerate developed tools for clinical teams to engage patients in the study design and execution stages of clinical development. These tools are expected to enable more effective patient engagement activities that may support the development of more patient-centric clinical study protocols and better understanding of the study participant experience.

P-PET Overview

- **The P-PET** is a set of deliverables that sponsors can leverage to engage with patients during the development of a clinical study. It is a way for sponsors to obtain direct input from patients early in the clinical study protocol design process.

- The P-PET includes:
  - User Guide
  - Resource Guide (Question Bank & Visual Aids—this guide)
  - Templates
The Resource Guide contains a list of sample questions and visual aids.

The sample questions in the question bank can be used during development of your patient engagement activity. The questions can be customized to fit your specific engagement activity.

Many of the visual aids are taken from the Patient Advisory Board template slide deck and are examples of how sections of the protocol could be presented to patients during an engagement activity. When considering alternative ways to present information keep the patient perspective in mind.

This tool was developed following the Common Protocol Template (CPT) as a guide so the sections and terminology are consistent between the Patient Protocol Engagement Toolkit (P-PET) and CPT (reference page 29).

### Each page in this Resource Guide is formatted as follows:

<table>
<thead>
<tr>
<th>Category</th>
<th>Guidance</th>
</tr>
</thead>
<tbody>
<tr>
<td>This section ties back to the CPT.</td>
<td>This section provides considerations for presenting the information to the patient.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Study Element</th>
</tr>
</thead>
<tbody>
<tr>
<td>This section ties back to the CPT.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Question Bank &amp; Prompts</th>
</tr>
</thead>
<tbody>
<tr>
<td>This section provides guidance for sponsors to develop a script to use when seeking inputs for a study from patients. It includes sample questions and prompts that can be used during the engagement. Choose the question(s) that best address your purpose.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Visual Aid</th>
</tr>
</thead>
<tbody>
<tr>
<td>The section includes patient-friendly slides (visual aids) with specific areas of focus and questions.</td>
</tr>
</tbody>
</table>
While reviewing these questions and visual aids, please remember:

- The use of “patient” throughout can refer to anyone speaking for or representing the patients’ voice, e.g., patients, caregivers, parents, patient advocates, patient advisors, or expert patients.

- 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.
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**Question Bank & Prompts**

- **Probing for general understanding about the study rationale.** Ask what would a "clinically meaningful improvement" mean from a patient’s perspective for their condition/disease.

- **Assess perceived benefit/risk for study participation**
  - From what you know so far about this study, what do you think it would be like to participate?
  - What do you think are the good things about participating and how important are these things?
  - What do you think are the not-so-good or bad things and how important are these things?
  - What are the potential barriers for your interest in participating in the study?
  - What aspects of the study design/rationale do you like?

- **Assess relative “importance” of factors to patients**
  - What could the researchers do to help assess the importance of factors from a patient’s perspective?

- **Assess unmet medical need**
  - What are the hardest things about living with this condition?
  - What do you want to do that you can’t do now?

- **If you could improve one thing about living with your condition, what would it be?**

- **What do you hope to get out of this clinical study? What improvements do you hope to see? What do you expect will happen?**

- **Please think about the current care and any other care that may be available for the patients condition. Is current care meeting all your expectations? If not, why?**

**Visual Aid**

- To facilitate the discussion, consider having patients draw pictures to depict their condition.
- Use symbols to communicate, when reasonable.
Category

- Schedule of Activities & Study Design

Guidance

- Ensure participants’ understanding of terminology, such as endpoints and objectives
- Outline rationale for selection of measures; explain if the measures are required by regulatory agencies, payers, etc.
- List options for measures
- Approaches: Get feedback on existing draft or start from beginning and co-create. Depends on established disease standards, team objectives, etc.

Question Bank & Prompts

- The most important measures for this study are <primary and key secondary objectives and endpoints> because <rationale>.
- How important are the <primary and key secondary endpoints> (to the target study population)?
- Are there other symptoms or other things about the condition that are more important?
- Would you suggest a different primary endpoint? If so, why?
- Would you suggest a different measure as the most important one?
- Would you suggest any other measures?

Visual Aid

- Ensure “primary and secondary endpoints” are clearly defined

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Study Design & Schedule of Activities

Category

- Schedule of Activities & Study Design

Study Element

- Study Design / Overall Design

Guidance

- Explain all relevant terminology (e.g., open-label extension, crossover design)
- Explain all steps of the study
- Show the consent process
- Idea: Have an illustrator represent all key steps of the clinical study journey as a roadmap
- Describe the clinical study journey for an individual participant and the study overall

Question Bank & Prompts

- What is your general feedback on the overall study design?
- What aspects of the study design might cause people to:
  - take an interest or not take an interest?
  - want to join or not want to join?
  - want to stay in or not stay in the study?
  - have a good experience or not with the study?
- What do you think about a <#>% chance to be assigned to placebo / drug y? What are your comments about this? What might help? (e.g., explore crossover design, open-label extension)
- The study does / does not have an opportunity for study participants to receive active drug at the end of the study. How important would having that opportunity be to you or someone with your condition?
- If there was an opportunity to change one thing about the overall study design, what would it be?
- How do you feel about the fact that the study participant and the doctor will not know what study intervention you/the patient would be getting? (However, consider explaining that the masking can be broken in case of emergency.) This is important because <rationale> / We want to keep this because <rationale>.
- A patient who participates in the study will be in the study for about <x to x months>. During this time the study participant would need to <explain required activities> / would not be able to <explain restricted activities>. What are your comments about this? Are there any changes that would make that better?
Category

- Schedule of Activities & Study Design

Study Element

- Schedule of Activities

Guidance

- Explain all relevant terminology
- Associate information with the “Study Design” (see above)
- Idea: Have an illustrator represent all key steps of the clinical study journey as a roadmap
- Use symbols to represent activities as practical
- Show videos, photos, or illustrations of study activities, if possible. Use symbols to represent activities, if possible. Call out any invasive procedures. Explain what lifestyle restrictions are required before or after any procedures, as this information might influence feedback about the Schedule of Activities.
- Estimate the chances that a patient might screen fail and address when discussing screen fail procedures
- Considerations for study designers:
  - Could patients be offered an option to complete the visit activities over multiple days?
  - What is the required or desired order of study activities? For example, it is common to recommend that patient-reported outcomes instruments be completed before any invasive procedures, and for investigational product to be administered as the last procedure; is this timing practical for patients?
  - Could study visits be coordinated around the patients’ regular visits?
  - For any invasive procedures conducted during screening, could those procedures occur after the non-invasive procedures that might exclude patients earlier (so that you are not exposing patients to invasive procedures when those patients might have already been excluded)? Consider the expected timing of availability of the results of those invasive procedures to support the investigator’s determination of eligibility.
  - Could the study utilize data from those invasive procedures that might have already been collected within x days prior to screening and avoid the need to conduct those procedures again during the study? The Protocol and ICF would need to address the possible use of these data.
The estimated duration of <visit x> would take <x hours> and <would need to start / end by x time>. Do you have any comments on this?

The activities [including invasive procedures] that would happen at <visit x> are <activity 1>, <activity 2>, and <activities 1 and 2 would need to be done within x time>. What comments do you have about having all these activities happen in one visit? How would you want these activities to happen (one long visit or 2 shorter visits, etc.)?

What parts of the visit schedule might cause people to not take an interest in this study or want to join the study or stay in the study / have a good experience with the study?

What visit schedule changes would you recommend? If we can’t change this, how could we make this better?

Based on the visit schedule are there any out-of-pocket costs one can anticipate based on participation in the study? Are these acceptable?

What support would be needed (e.g., transportation to the site, childcare arrangements, adult care arrangements)?

Would it make a difference if any of the visits could be done virtually (e.g., on computer, video conference, or telephone) or by having a healthcare professional visit at home? Visits at local pharmacy? Drug dispensed here or sent to home? Combination of home and office visits?

Look at the screening-specific procedures. How willing would a patient be to complete these procedures without knowing that they will definitely be in the study? If there was an opportunity to get rid of one of the screening procedures, which would it be?
Study Population

Category

- Study Population

Guidance

- Explain all relevant terminology
- Describe the target population at a high level early in the discussion; address study population details later
- Divide criteria into categories (e.g., age, medications)

Study Element

- Inclusion & Exclusion Criteria

Question Bank & Prompts

- Do you think that the requirements represent people with this condition?
- What is your feedback about the criteria in general? Why?
- How might these criteria affect someone’s decision to join the study?
- How easy would it be for someone to get this information on their own to check their eligibility without talking to their healthcare provider (doctor, nurse)?
- What would you want to change for each criterion? Are there any requirements you would want to change? If so, which do you think are most important?
- What do you think of the <age, weight, other> requirements?
- What requirements might prevent patients with <condition> from being able to be in the study?
- Are any of the restrictions about other therapies (past or present) a problem?
- Do you see any problems with any of the testing that would need to be done to make sure people with <condition> could join the study?

Visual Aid

Eligibility Criteria

- Inclusion Criteria:
  - X - Y years of age
  - Has a history of this condition for at least the past 12 months
  - If took medication e, took less than x amount in the past 3 months
  - Willing to stop taking medications e, f, and g ("washout")
  - Willing to use contraception, through 12 weeks after the last dose of study drug

- Exclusion Criteria:
  - Has less than x value on a laboratory test involving several blood draws
  - Does not have a history of conditions 2, 3, or 4 over the past 5 years
  - Did not have a good response to medications a or b in the past 3 months
  - Did not take medications c or d for any condition in the past 6 months
**Study Population**

<table>
<thead>
<tr>
<th>Category</th>
<th>Guidance</th>
</tr>
</thead>
<tbody>
<tr>
<td>Study Population</td>
<td>Explain all relevant terminology</td>
</tr>
<tr>
<td></td>
<td>Use images that will help patients envision how any restrictions might affect them day-to-day</td>
</tr>
</tbody>
</table>

| Study Element | Lifestyle considerations |

**Question Bank & Prompts**

- Thinking about how people with `<condition>` typically live their life, how might these restrictions affect the patient, the caregiver (as applicable), and the family? Socially? Emotionally? Physically?
- Would these lifestyle considerations meaningfully increase patient burden?
- Would these lifestyle considerations stop you or someone with this condition from:
  - Joining the study?
  - Staying in the study (not stopping the study early)?
  - Following all the study requests?
- Which of the restrictions would be hard to follow?
- What would help to follow the restrictions (for example, reminders, counseling, something else)?
- If there were an opportunity to change any of the restrictions, which would you change? Which do you think would be the most important of these changes?

**Visual Aid**

- Do not eat more than a full-full size of food within 3 hours before or 3 hours after receiving the study drug.
- Do not eat or drink caffeine (such as espresso beans, coffee, tea, cola drinks) or alcohol from the start of screening until 30 days after the last dose of study drug.
- If you already use nicotine products (such as cigarettes, chewing tobacco, nicotine patches), you can use them as you normally do throughout the study. If you plan to change how you use nicotine, please tell the study staff.
- Do not do heavy exercise (such as running or weight-lifting) for 6 hours before and 12 hours after receiving the study drug. You can do light exercise (such as walking) during this time.
Study Intervention

**Category**
- Study Intervention

**Study Element**
- Study Intervention(s) Administered

**Guidance**
- Explain all relevant terminology
- Show actual packaging when possible (e.g., package insert if for patient home use)
- Use role play if that might help with understanding
- Illustrate all aspects of how full “compliance” is defined for the study
- Demonstrate any special handling and storage procedures
- The word ‘intervention’ may require clarification when engaging with patients
- If study medication will be given in conjunction with standard of care, ensure the concept of standard of care is well understood by patients

**Question Bank & Prompts**

- What about the study intervention might make someone with this condition want to join or to stay in the study or make it hard to complete all the visits as scheduled?
- What do you think about <method of administration - e.g., infusion over 30 minutes> in <setting, e.g., hospital> every <frequency> for <duration>?
- What do you think about the possibility that the dosing might change if there are side effects?
- Are the instructions for dosing clear to follow? Any suggestions for changes?
- If you could change one aspect of the study intervention, what would it be?
- Do you see any problems with any of the study interventions?
- What do you think about how/where the intervention is given, and how long it takes (on any given day and over the entire study)?
- What support would someone need to be sure that they could follow the administration requirements? Support might include things like reminders.
- Check for understanding for any training materials and process for reporting adverse events.
- Do you think there is anything that would cause someone with this condition to quit the study before the end?
- Do you think there are any problems with how someone with this condition would receive/transport/need to store the intervention at home given (e.g., size of the packaging, need for refrigeration)?

**Visual Aid**

- **Study Treatments Information**
  - Study drug X / Placebo is given by infusion every 7 days at the hospital. Each infusion will take 15-30 minutes.
  - Study drug Y / Placebo is taken at home every day with 4 pills, twice per day
  - If you miss a dose on a day, do not take more pills the next day
  - You will be treated for 12 months. If you have certain side effects the study doctor will modify the dose.

* If study medication will be given in conjunction with standard of care this should be depicted in your visual aid
Study Intervention

**Category**
- Study Intervention

**Guidance**
- Explain all relevant terminology
- Check for possibility to have test devices available for hands on training
- Check for understanding for any training materials and process for reporting adverse events and device/packaging fail event

**Study Element**
- Medical Devices

**Question Bank & Prompts**
- What do you think about the medical device as described?
- Is the packaging user-friendly?
- Is the labeling clear, or is it confusing in any way? If so, what would make more sense?
- Are the instructions on how to use the device clear? Any suggestions for improvement?
- What do you think about the following in using the device?:
  - carrying it where the study participant needs to go
  - the special equipment needed
  - how to store it
  - the carrying case
  - what people might think
- Can you tell me what you understand about how to use the device? How to report any side effect or if the device does not work properly?

**Visual Aid**
- Show actual device when possible
Question Bank & Prompts

- How would you feel about the possibility that the dose might have to be skipped, delayed, increased or decreased? If it had to be stopped for a side effect, how would the study participant feel about taking it again (knowing that a doctor would be carefully monitoring them)?

Visual Aid

- Consider a flow chart to explain and illustrate dose modification
## Study Intervention

<table>
<thead>
<tr>
<th>Category</th>
<th>Guidance</th>
</tr>
</thead>
<tbody>
<tr>
<td>Study Intervention</td>
<td>Explain all relevant terminology</td>
</tr>
</tbody>
</table>

### Study Element

- Concomitant Therapy

### Question Bank & Prompts
- What comments do you have about the rescue / concurrent / prohibited medications?
- To what extent would this affect someone with this condition?
- What do you think of other medicines that might need to be used?
- Are there any concerns about other medications that could or would also be used?
- Are there any concerns about medications that are not allowed during the study?
- Are these concerns for patients with this condition and/or do these concerns affect you personally?

### Visual Aid
- N/A
# Discontinuation of Study Intervention and Participant Discontinuation / Withdrawal

<table>
<thead>
<tr>
<th>Category</th>
<th>Guidance</th>
</tr>
</thead>
<tbody>
<tr>
<td>Discontinuation of Study Intervention and Participant Discontinuation / Withdrawal</td>
<td>➢ Define again the “study intervention”&lt;br&gt;➢ Define the reasons why patients may need to discontinue study medication&lt;br&gt;➢ Show any post-intervention / discontinuation / withdrawal activities as a part of the overall study roadmap</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Study Element</th>
<th>Question Bank &amp; Prompts</th>
</tr>
</thead>
<tbody>
<tr>
<td>Discontinuation of Study Intervention</td>
<td>➢ What reaction do you have regarding the reasons the sponsor may request that treatment intervention be stopped on a patient in the study?&lt;br&gt;➢ How might someone with the same condition feel about staying in the study for follow-up even after the treatment intervention has ended (early or as scheduled)?</td>
</tr>
</tbody>
</table>

**Visual Aid**

- N/A
Discontinuation of Study Intervention and Participant Discontinuation / Withdrawal

**Category**
- Discontinuation of Study Intervention and Participant Discontinuation / Withdrawal

**Guidance**
- Define again the “study intervention”
- Show any post-intervention / discontinuation / withdrawal activities as a part of the overall study roadmap

**Study Element**
- Lost to Follow Up

**Question Bank & Prompts**
- What would be the best ways to stay in contact with participants in the study? What would help keep them involved and help them continue to be followed?
- What might cause someone to not continue to be followed? What could help with this?

**Visual Aid**
- N/A
### Study Assessment and Procedures

**Category**
- Study Assessment and Procedures

**Guidance**
- Reference the Schedule of Activities when describing the assessments

**Study Element**
- Efficacy Assessments, Safety Assessments, Adverse Events (AEs) and Serious Adverse Events (SAEs)

### Question Bank & Prompts

- What do you think about how often `<safety, efficacy, other>` is measured? Is that the right timing for someone with this condition?
- What do you think about how often `<ECG, blood draws, urine samples, other>` is measured? Is that the right timing for someone with this condition?
- Would it be helpful if any of this could be done over the phone or computer, or by having a healthcare professional visit at home?
- Are the things being measured important to you or to someone with the same condition?
- What do you think about how one might report some of this information `<for example, electronic or paper diary>`?
- What do you think about how all side effects or other symptoms will be reported to the researchers? Do you recommend any changes?

### Visual Aid

**Study schedule.**

<table>
<thead>
<tr>
<th>Visit Period</th>
<th>Visit Number</th>
<th>Pre-Study</th>
<th>Baseline</th>
<th>Post-Treatment</th>
<th>Pre-Treatment</th>
<th>Baseline</th>
<th>Post-Treatment</th>
</tr>
</thead>
<tbody>
<tr>
<td>Scheduled Week</td>
<td>0 to 4</td>
<td>A</td>
<td>A</td>
<td>A</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Informed Consent</td>
<td></td>
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</tr>
<tr>
<td>Medical History</td>
<td></td>
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<tr>
<td>Review Medications You’re Taking</td>
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<tr>
<td>Physical Exam</td>
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<tr>
<td>Vital Signs</td>
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<tr>
<td>Electrocardiogram (ECG)</td>
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<tr>
<td>Review of Diary</td>
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<td>Questionnaire (if applicable)</td>
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<tr>
<td>Urine Pregnancy Test (if applicable)</td>
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<tr>
<td>Study Drug Disposition</td>
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</tr>
</tbody>
</table>
Question Bank & Prompts

• What do you think about how often samples are taken for drug levels or for other measures? Is that a problem in any way for someone with the same condition?
• Do you see any problems with any of the longer visits that are needed to give these samples? What could make that better?
Study Assessment and Procedures

**Category**
- Study Assessment and Procedures

**Study Element**
- Genetics, Biomarkers

**Guidance**
- Explain all relevant terminology
- Reference the Schedule of Activities when describing the assessments
- Consider potential impacts such as health insurance, withdrawal of consent, privacy consent

**Question Bank & Prompts**
- Do you understand the reasons for the testing? Do you have any concerns?
- What is your understanding of what this testing is?
- If you have any concerns, what other information would you want to know about these tests?

**Visual Aid**

The following table lists an example that could help you communicate to the patient what you can expect at each visit:

<table>
<thead>
<tr>
<th>Visit Period</th>
<th>Prestudy</th>
<th>Baseline</th>
<th>Treatment 1</th>
<th>Treatment 2</th>
<th>Treatment 3</th>
<th>Discontinuation</th>
<th>Post-treatment</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>1-3</td>
<td>4-6</td>
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<tr>
<td>Informed Consent</td>
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<tr>
<td>Medical History</td>
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<tr>
<td>Review Medications You’re Taking</td>
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<tr>
<td>Physical Exam</td>
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<tr>
<td>Visual Acuity</td>
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<tr>
<td>Electroencephalogram (EEG)</td>
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</tr>
<tr>
<td>Review of Labs</td>
<td></td>
<td></td>
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Confidential
Study Assessment and Procedures

- **Category**
  - Study Assessment and Procedures

- **Study Element**
  - Health Economics or Medical Resource Utilization and Health Economics

**Question Bank & Prompts**

- Do you have any comments on these measurements and how they are collected? Is there anything you would change?
- Do you feel the study participant would be willing to complete all the questionnaires given the time it takes to complete over the course of the study?
- Is the time investment needed to complete the tool reasonable for what the tool is measuring?
- Is there a method of delivery (paper or ePRO/electronic) for the questionnaires that you feel is better?

**Visual Aid**

The table is an example that could help you communicate to the patient what can be expected at each visit:

<table>
<thead>
<tr>
<th>Visit Period</th>
<th>Pre-study</th>
<th>Baseline</th>
<th>Treatment 1</th>
<th>Treatment 2</th>
<th>Treatment 3</th>
<th>Treatment 4</th>
<th>Discontinuation</th>
<th>Post-treatment</th>
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<td>Week 6</td>
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<td>Review Medications You’re Taking</td>
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</table>
General Topics

Category

- General Topics

Study Element

- Technology
- Reference to Study Operational Plans

Guidance

- Explain all relevant terminology
- Show actual technologies when possible
- Consider using prototypes and co-creating solutions with patients
- Reference the Schedule of Activities when describing when and how the technologies might be used

Question Bank & Prompts

- Are there any technologies that would make things easier?
- What technology would help people with:
  - Joining the study?
  - Staying in the study (not stopping the study early)?
  - Following all the study requests?
- What challenges would someone have when trying to use technology?
- What support would someone need to be sure that they could use the technology?
- What options might people want?
- If you could have one technology solution for this study, what would it be?
- Would there be preference to use personal smart phone or a handheld device that is given to use only as a part of the study?
- What technologies do you use to monitor someone with this condition? How do you expect this technology to help?
- Is there another technology or way to use current technology to help someone with this condition?
- If there were a technology used in the study, do you think someone with this condition would be willing to use it?
- Would this condition in any way make it hard to use that or another technology?
- Will the technology be an aid, or do you foresee any problems?
- Are there any other technologies that could help in this study (e.g., apps, wearables, study website, SMS text reminders)?
- Are you aware of someone with this condition already using any other technologies (e.g., are you/the patient regularly monitoring biometrics, logging symptoms) and if so, would their be willingness to share this information with the study doctor as part of the study?

Visual Aid

Think about how you use technology each day. If you were participating, or thinking about participating, in this study, which of these technologies (if any) would you like to use for each of the following activities?

- Learning about drug development and clinical research / studies that are enrolling / this study
- Sharing information about this study with family/friends
- Remembering to attend a study visit
- Remembering to complete study activities when you are not at the site
- Reporting side effects
- Organizing transportation to/from the site
- Scheduling childcare or other household support so you can attend a study visit
- Reminding to take your study drug
- Communicating with the study staff / for routine messages
- Learning about the status of the study
- Receiving compensation for travel, parking, and other study-related expenses
- Being thanked for your participation in the study
- Verifying your own data
- Sharing your own data with your treating / usual primary care physician or other healthcare provider
- Verifying the results of the study or
- Learning about new studies that might interest you or a family member / friend
- Providing your advice about a study

Confidential
### General Topics

<table>
<thead>
<tr>
<th>Category</th>
<th>Guidance</th>
</tr>
</thead>
<tbody>
<tr>
<td>General Topics</td>
<td>There may be other questions relevant to patient participation in the study</td>
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</table>

| Study Element     | N/A                                                                        |

### Question Bank & Prompts

- For an element that a patient thinks significantly affects participants: What is the best way to help patients understand `<element>`.
- What are your top 3 concerns? What parts of the study most interest / excite you? Write down privately first, then feel free to share with group…
- What problems, if any, do you see with personal health insurance and this study for someone with this condition?
Appendix
Objectives:
What the study is testing
Clinical studies are used to test important things, like "Is drug x this much better than placebo and drug y for the treatment of a condition?" An objective is used to see if a test was passed. An objective might be to look at improvement in a symptom. "Primary" Objective = The most important objective. There can be 20 or more other objectives for a study.

Endpoints:
How the study will measure what it is testing
An endpoint is something that is measured to check if an objective was met. An endpoint might be a score on a questionnaire that is used to look for changes in a symptom. It could also be a laboratory value, signs of a condition, quality of life, and length of life. There can be more than 1 endpoint for each objective.

Study Design:
The study design is how the study is set up to test the important things, like the effect of drug X compared to placebo and drug y on the changes in severity of symptom 1. It includes what the main study drug will be compared to (like placebo and/or drug y), for how long, with how many patients, and who will know what the patients are getting in the study.

Different study designs have names like "parallel-group" and "crossover" The design should help researchers know that the study will give them information that will really address the objectives and not different things. Example of a 'bad' design: Letting patients know that they are getting drug x and not placebo. This could cause patients to say that the severity of symptom 1 is less than what the drug x is really doing on its own.
Definitions

Study Interventions:
Study intervention are the drugs/devices that will be used in the study. A study intervention can be the investigational drug or device (not yet on the market for the condition) or a drug that is prescribed routinely for this condition. It can also be a placebo. The study intervention will be administered over a certain period of time to see how they work for people.
It is important to know that:
• People might be assigned to study interventions by chance (randomization)
• People might not know which intervention they will receive or are receiving (blinding)

Eligibility Criteria:
Eligibility criteria are “rules” about the types of people who can enter a study. The same rules are applied to everyone and are based on factors like age, medical and psychological conditions, and medications taken.
  “Inclusion criteria” = OK to enter study
  “Exclusion criteria” = Not OK to enter study
It’s important to let many different types of people into a study so that we can better know how the drug will work for them. However, the rules are needed for a study so that people don’t get into the study who:
• Are likely to be harmed by the study drug or are unlikely to be helped by the study drug
• Have other conditions or symptoms or are taking treatments that would make it hard to know how the study drug is working
**Definitions**

**Lifestyle Considerations:**
Studies might have lists of things that you should do (start or keep doing) or should not do in your everyday life at specific times or for the entire time while you are taking part in the study. These things can be activities (such as driving), eating certain foods (such as grapefruit), drinking certain beverages (such as alcohol), or taking in certain substances (such as nicotine). The things that are listed usually affect how the study drug works in the body or might make it hard to know what the drug does, or they may be things which will hurt you while you are in the study. For example, grapefruit contains a substance that can stop certain drugs from working. This is why studies of those drugs would ask study participants to not eat grapefruit or drink grapefruit juice.

**Technology:**
Smartphones, personal digital assistants, tablets, laptops, desktop computers, smart watches, activity or sleep trackers, and specialty devices (such as glucose monitors), and using websites, apps, video chat, etc.
The Common Protocol Template (CPT) is a streamlined approach to the format and content of clinical study protocols. It aims to ease interpretation by the study sites and global regulatory authorities while enabling downstream automation of many clinical processes and aligning to industry data standards. The CPT includes a common structure, common text, and regulator-accepted endpoint definitions that may be used across protocols with little to no editing at the discretion of the user.

The CPT can be found at: [http://www.transceleratebiopharmainc.com/assets/common-protocol-template/](http://www.transceleratebiopharmainc.com/assets/common-protocol-template/)

<table>
<thead>
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<th>Category</th>
<th>Study Element</th>
<th>Reference to CPT Template Section(s)</th>
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<td>Health Economics or Medical Resource Utilization</td>
<td>8.9</td>
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</table>
Other References

- **TransCelerate Patient Protocol Engagement Toolkit**
  - User Guide
  - Resource Guide (Question Bank & Visual Aids-this guide)
  - Templates
- **TransCelerate Patient Technology Tools**: [https://www.transceleratebiopharmainc.com/patient-technology-assets/](https://www.transceleratebiopharmainc.com/patient-technology-assets/)
- **EUPATI** [https://www.eupati.eu/](https://www.eupati.eu/)
- **IMI-Paradigm** [http://imi-paradigm.eu/](http://imi-paradigm.eu/)
- **Patient Focused Medicines Development** [https://involvement-mapping.patientfocusedmedicine.org/initiatives](https://involvement-mapping.patientfocusedmedicine.org/initiatives)
- **Patient Focused Medicines Development**: *Reasonable agreements between patient advocates and pharmaceutical companies* [https://patientfocusedmedicine.org/reasonable-legal-agreements/](https://patientfocusedmedicine.org/reasonable-legal-agreements/)