Patient Technology Implementation Framework

Introduction

Patient Technologies (PT) include any digital technology with which patients interact to engage in clinical trial activities. The use of PT in the clinical trial space is growing, but is not yet a wide-spread practice. Organizational challenges to full-scale implementation of PT in trials persist, including:

- High barriers to entry and steep learning curves
- Limited alignment and knowledge-sharing between stakeholders
- Lack of organizational PT strategies or accommodating processes
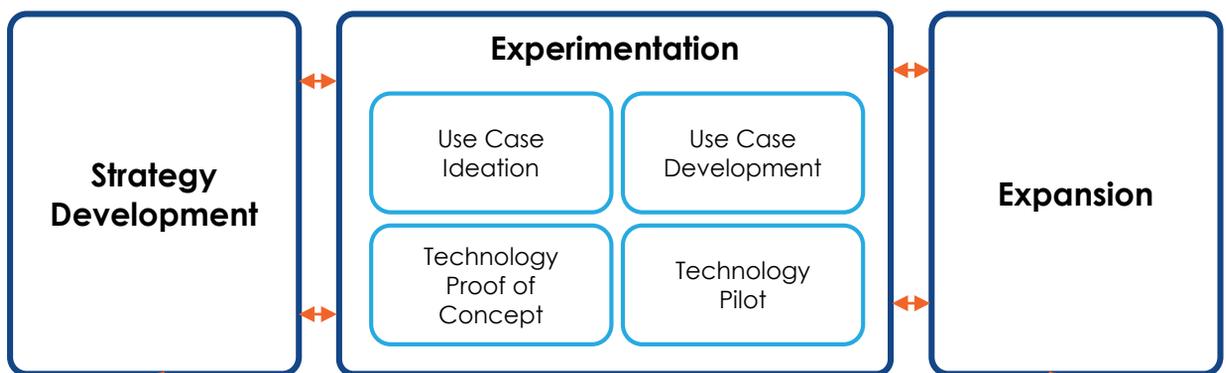- Limited understanding of stakeholder needs and experiences

The Patient Technology Implementation Framework was designed specifically to address these challenges. It will help clinical trial teams within a particular company to develop a holistic approach to PT, with the organization's long-term goals in mind.

The framework provides potential approaches for:
- ➢ **Getting started or moving forward** with PT implementation
- ➢ **Identifying important considerations** early in the program design process
- ➢ **Incorporating an iterative, rapid-learning approach** to PT selection and implementation
- ➢ **Considering patient and site perspectives** during program design
- ➢ **Utilizing additional resources** that may offer insights or support during PT program design

Using the Patient Technology Implementation Framework

Select a stage of the framework to get started or the links below to learn more

Strategy Development

Experimentation
- Use Case Ideation
- Use Case Development
- Technology Proof of Concept
- Technology Pilot

Expansion

User Guide & Frequently Asked Questions
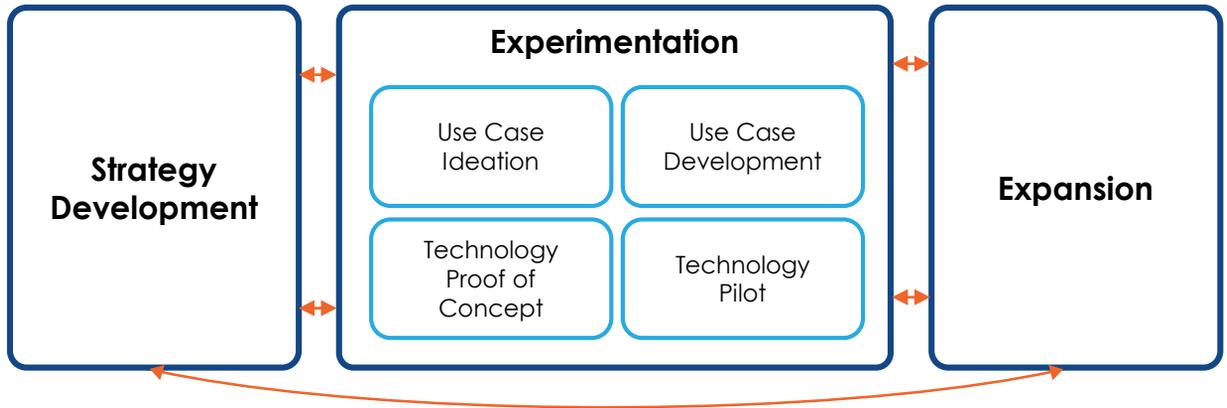
Tools & Resources

Stage Selection Guide
Determine when to progress to each stage of the framework

Patient Technology Implementation Framework
Stage Selection Guide

The *theoretical* flow of the framework proceeds from strategy development to use case ideation, use case development, technology proof of concept, pilot, and expansion. In reality, programs may require more fluid or iterative progression through the stages. **Therefore, teams should progress or revisit the stages as needed based on the maturity and status of their PT program.**

Strategy Development

Experimentation
- Use Case Ideation
- Use Case Development
- Technology Proof of Concept
- Technology Pilot

Expansion

Stage	Stage Definition	Incorporate this stage to…
Strategy Development	Develop a company-level strategy that outlines an internal approach to Patient Technologies for specific technology types, therapeutic areas, or teams/functions, with defined program goals	• Develop a PT strategy for the first time • Refine an existing PT strategy
Use Case Ideation	Identify potential use cases, technologies, and endpoints for additional development, and identify implementation approaches for PT within a study or related series of studies	• Shape a novel PT program • Consider new use cases for a previously used PT • Refine a previous use case
Use Case Development	Conduct additional research and scoping to further develop a use case and PT experimental plan for a study or related series of studies. Define the goals and requirements, and determine how to approach implementation to achieve a scalable solution.	• Develop a novel PT use case • Refine or expand an existing PT use case
Technology Proof of Concept	Conduct rapid, iterative testing of the PT to understand functionality, ensure fit for purpose, mitigate risks, and determine readiness for pilot. Address unknowns or risks associated with the PT use case *outside of a clinical trial setting*.	• Compare candidate technologies • Characterize a selected technology • Follow-up on past learnings
Technology Pilot	Implement the PT experimentally in the desired use case within a clinical trial or study. Gather experiential data, assess real-world feasibility of the PT, develop learnings to inform future work, and determine readiness for expansion.	• Assess a PT for the first time • Conduct subsequent PT pilots • Test a PT in a new use case
Expansion	Develop a strategy for the scaled implementation of PT across studies, programs, therapeutic areas, and geographies, based on learnings from each of the previous stages	• Scale a PT that has been piloted previously

Patient Technology Implementation Framework
Strategy Development

Stage Definition

Develop a company-level strategy that outlines an internal approach to Patient Technologies for specific technology types, therapeutic areas, or teams/functions, with defined program goals.

Approach

Determine goals for scaled PT at the company, program, therapeutic area, and technology level
- Evaluate the landscape (Landscapes may include company goals, pipeline or strategy, patient priorities, academic research, competitive efforts, technology offerings, regulatory considerations, internal knowledge, precedence, site sentiment, etc.)
- Identify the organization's primary objectives for implementing PT. Clearly define the challenge the PT should address and the benefit it should bring. Ensure they are in line with organization strategy
- Clearly define desired benefits to patients, study programs, or other stakeholders. Engage patients or other stakeholders to establish goals, priorities, and value propositions as needed.
- Identify key risks, knowns, and unknowns

Develop business case and/or a business case development plan at the program level
- Outline value proposition, risks, and costs. Consider current status and future goals.
- Identify unknowns and data required to develop a business case
- Define measurable endpoints and KPIs with respect to overall program goals
- Define metrics that teams can use in individual studies to contribute to the overall business case
- Develop plan to track and refine business case over time

Develop implementation strategy at the program level
- Evaluate potential impacts of the PT program on study design(e.g., costs, sourcing, timing, etc.)
- Identify target segments for use of PT (e.g., therapeutic areas, patient populations, geographic regions). Identify stakeholders and the engagements or inputs required with each.
- Identify challenges or risks to adopting PT in this program (e.g., unknowns, risks, costs, timing, patient and other stakeholder burdens, resource gaps, organizational barriers). Engage study teams and other stakeholders as applicable.
- Develop a plan to address risks and unknowns through additional research, collaborations, proof of concept development, studies, or through other engagement activities (e.g., regulatory or patient engagement).
- Develop guidelines for study teams to coordinate PT implementation & pilot design across studies.

Patient Technology Implementation Framework

Use Case Ideation

Stage Definition

Identify potential use cases, technologies, and endpoints for additional development, and identify implementation approaches for PT within a study or related series of studies.

Approach

Brainstorm problem statements, research questions, and desired value
- ➤ What do you want to achieve, why do you want to achieve it, and why/how can PT help you achieve it?
- ➤ Consider goals and impacts from an organizational perspective, program perspective, patient perspective, etc.

Understand the Landscape – Both internal and external
- ➤ Past Work & Organizational Landscape
  - ▪ What has been done in the past, either internally or externally in industry or academia?
  - ▪ What were the reasons for success and failure?
  - ▪ What is known about patient needs, priorities, and burdens? What does meaningful change look like to these patients? Pursue engagements as necessary.
  - ▪ What experiences/capabilities exist within the company?
  - ▪ Identify learnings from past work and gaps (e.g., processes, resources, expertise) that must still be addressed. Should these gaps be filled internally, or should they be outsourced?
- ➤ Technical Landscape
  - ▪ What tools, technologies , and vendors exist? What is technologically feasible?
  - ▪ What internal development or infrastructure might be required?
- ➤ Strategic Alignment & Organizational Support
  - ▪ How could a PT program fit into the organization's strategy and broader body of PT work?
  - ▪ Who are the internal and external advocates and opposition? What motivates them?
  - ▪ What external initiatives or organizations impact or align with your goals?

Brainstorm solutions, use cases, and endpoints, solutions, and technologies
- ➤ This brainstorming activity should focus primarily on potential use cases, endpoints, and approaches, rather than specific technology options. In general, this stage and its accompanying activities usually precede the selection of a technology, to ensure the technology is fit for purpose.

──────────── **Refine, Pivot, or Proceed:** ────────────

Assess the use cases for theoretical feasibility and alignment with strategy. Based on the landscape assessment, is there potential for any of your use cases to bring the desired value (to the company, to science, to the patients, or otherwise) and address the problem statement you laid out? If so, select one or more of the most promising use cases for additional research and further development.

Patient Technology Implementation Framework
Use Case Development

Stage Definition

Conduct additional research and scoping to further develop a use case and PT experimental plan for a study or related series of studies. Define the goals and requirements for the PT use case, and determine how to approach implementation to achieve a scalable solution.

Approach

Confirm or refine the problem statement, research question, and desired value
- Outline objectives for the PT use case based on the research question, organizational goals and strategy, and the desired benefits of the PT

Identify groups and capabilities required to support the PT program
- Working with PT may require infrastructure or additional team members compared to traditional clinical trial design. Determine what additional expertise or support you may need (e.g., increased IT support, technology specialists, new data management or analytical capabilities, market research or user experience research capabilities), and engage with appropriate groups early in the development process.

Identify and characterize internal and external stakeholders
- Identify target users of the PT. Identify and characterize target patient demographics, therapeutic areas, geographic regions, trial phases, etc. As needed, pursue patient or caregiver engagements to further develop understanding of needs and priorities.
- Identify other stakeholders impacted by the PT use case. This may include internal stakeholders, sites, CROs, caregivers, or others.
- Consider regulatory implications of the PT and, if appropriate, engage relevant health authorities for feedback.
- Identify the drivers and barriers experienced by critical users and stakeholders, and how they may affect PT implementation. As appropriate, engage with these stakeholders to gather insights.

Identify requirements for the PT and the use case (in pilot and at scale)
- Identify endpoints, functionality, features, and data streams that will be required or desired to achieve study and program goals
- Identify novel considerations that may impact study design or support. This may include user acceptance, IT infrastructure, data management, validation, translation, or other considerations.
- Incorporate perspectives and needs from applicable stakeholders to generate additional requirements. Perspectives can be gathered from engagements, research, past learnings, or published work.

Identify potential technologies
- Identify potential technologies, vendors, or technology development options that appear fit-for-purpose and warrant further exploration

Patient Technology Implementation Framework
Use Case Development

Approach (Continued)

Identify questions, gaps, unknowns, and required research
- Identify assumptions or unknowns with regard to technical feasibility, value proposition, usability, stakeholder perspectives, regulatory considerations, implementation and scalability. Identify those that must be addressed to meet study or program requirements
- Identify potential challenges associated with scaling the PT from a pilot to more mainstream use
- Identify challenges or risks to adopting PT in this program (e.g., unknowns, risks, costs, timing, stakeholder burdens) using a risk assessment tool such as FMEA. Engage the technology's users and stakeholders as applicable.

Develop a PT Experimentation Plan
- Develop a plan for addressing unknowns, mitigating risks, and establishing the value proposition for the PT, in preparation for both pilots and scaled solutions
- Identify which critical unknowns can be addressed through additional research, engagement with stakeholders, proof of concept testing (as described in the Technology Proof of Concept Stage), or technology pilots (as described in the Technology Pilot Stage).
- For unknowns that can be addressed through technology proof of concept testing, develop methods and approaches to conduct such testing. Set up a process to capture learnings, and use learnings to improve future testing or piloting activities.
- Define success criteria for each phase of testing
- Define backup plans, in case PT does not meet desired success criteria

──────────── **Refine, Pivot, or Proceed:** ────────────

Based on what you learned during landscaping, research, and planning, re-evaluate the use case's feasibility and alignment with strategy. Make go/no-go decisions, pivots, or adjustments as appropriate. After each decision, refine the use case with new landscaping, interviews, risk assessments, etc.

Patient Technology Implementation Framework
Technology Proof of Concept

Stage Definition

Conduct rapid, iterative testing of the PT to understand functionality, ensure fit for purpose, mitigate risks, and determine readiness for pilot. Address unknowns or risks associated with the PT use case *outside of a clinical trial setting*.

Approach

Based on the PT Experimentation Plan compiled in the Use Case Development Stage, it may be beneficial to conduct a series of Technology Proofs of Concept to inform technology selection, technology design, pilot design, and scaled implementation. This testing can also be conducted on any training or support materials required for use of the technology. Such assessments should provide high-level or directional information about a PT in the shortest time and at the lowest cost possible.

For the purposes of this framework, 'Technology Proof of Concept' includes tests or assessments of a PT that occur outside of a clinical , study, or trial setting. Experimental implementation of a PT in a clinical setting or through studies or trials are categorized as 'Technology Pilots.'

Types of Proof of Concepts
➢ Gather directional feedback from patients, sites, caregivers, or other stakeholders
  ▪ **Methods:** Examples include surveys, interviews, advisory boards, market research techniques, and meta-analyses or reviews of past work.
  ▪ **Goals:** Collect guidance or perspectives on direction, privacy, burden, and value; identify factors to consider in future testing; identify health-related factors that may impact PT implementation; etc.

➢ User experience (UX) testing
  ▪ **Methods:** Examples include usability testing through interviews, field tests, expert reviews, employee volunteers, etc.
  ▪ **Goals:** Understand user experience and acceptance of the technology; identify specific needs for training or tech support; identify points of confusion or potential opportunities for misuse of the technology; identify issues or required updates of the technology and user interface, etc.

➢ Technical or verification testing of PT
  ▪ **Methods:** When appropriate, technical assessments can be conducted as a series of rapid tests or experiments that enable technology characterization. It is often valuable to conduct such testing internally to ensure flexibility, develop institutional learning, and gain hands-on experience with the technology.
  ▪ **Goals:** Understand the robustness, accuracy, and maturity of a technology. Assessments may test functionality, connectivity, durability, data quality, accuracy, precision, noise, etc. If more than one technology is being used, ensure interactions between the technologies occur as planned.

Patient Technology Implementation Framework
Technology Proof of Concept

Approach (Continued)

Types of Proof of Concept (Continued)

➢ <u>Analytical Proof of Concept</u>
  ▪ Assess the feasibility of using certain methods, sampling techniques, or endpoints using available datasets from similar technologies. Run proof of concept analyses on these datasets to help refine the direction of PT selection and design, as well as analytical plan development

➢ <u>Mock studies or 'dry-runs' of a PT protocol</u>
  ▪ Simulate the implementation of a PT in an internal or non-trial setting to identify and address potential issues prior to implementation in a pilot.

What if Proof of Concept testing is not feasible?

➢ When testing is not feasible for a specific unknown, identify what will not be tested internally and pursue discussions with the technology's providers, subject matter experts, or others to assess these features of the PT.

Document Learnings

➢ Capture both positive and negative insights and use them to refine, improve, or adapt future technology experimentation. Ensure that learnings are shared and accessible to other teams.

Understand the Limitations

➢ Technology proof of concepts often cannot provide a comprehensive assessment of a technology. Rather, they provide directional insights rapidly to enable better and faster decision-making during technology assessment and design.

➢ Care should be taken to structure proof of concepts in such a way that they answer very specific questions and minimize 'false failures.' Since a technology's full suite of features and support may not be implemented in a given proof of concept, an apparent failure of the technology may in fact be due to overly broad or ambiguous proof of concept design. Clearly define success or failure criteria of these assessments. If a technology does not meet success criteria, first ask 'Can this issue be addressed?' before discontinuing the use case or technology.

━━━━━ **Refine, Pivot, or Proceed:** ━━━━━

Based on what was learned during each assessment, re-consider the feasibility and alignment with strategy for the use case and technology selection(s). Make go/no-go decisions, pivots, or adjustments as appropriate. After each decision, refine the PT Experimentation Plan with new data, metrics, questions or hypotheses. Conduct additional follow-up testing or re-visit technology options if necessary.

Patient Technology Implementation Framework

Technology Pilot

Stage Definition

Implement the PT experimentally in the desired use case within a clinical trial or study. Gather experiential data, assess real-world feasibility of the PT, develop learnings to inform future work, and determine readiness for expansion.

Approach

Defining 'Technology Pilot'
A technology pilot should generate evidence supporting the feasibility of the PT for scaled use, and should involve implementation of the PT *in a study or trial* with a targeted population of end users. When developing a pilot, teams should ensure continued alignment with the strategic goals for the technology.

Pilots can be used to validate the technology in a specific use-case, assess real-world functionality or adherence, test and refine training procedures, collect data for algorithm development, or support the development of a business case. Pilots may focus on specific aspects of implementation, or may test implementation of the PT comprehensively as it is desired to be used at scale.

Types of Pilots (Not mutually exclusive)
- <u>Validation Study</u> – A study to prove that the PT is accurately measuring the outcomes it was intended to measure according to GxP requirements. Some PT may require validation for new use cases, implementation methods, or patient populations.
- <u>Equivalency Study</u> – A study to prove that the endpoints measured by the PT are equivalent to the endpoints measured by traditional means.
- <u>Feasibility Study</u> – A pre-clinical or clinical technology study conducted with end-users outside of an interventional drug trial, designed to assess the feasibility of an implementation strategy.
- <u>Modified or In-Clinic Technology Assessment</u> – In some cases, the PT is intended to be used in a home setting but questions about the use case or the technology's validity remain. In these cases, the PT may be used initially during a clinic visit to generate supporting information.
- <u>Experimental or Limited Implementation in a Clinical Trial</u> – A pilot could be run as a standalone trial or as an exploratory arm of a larger a Phase 1, 2, 3, or 4 trial (potentially with a limited number of sites or in a sub-set of a trial population). Any data or endpoints generated by the PT could be categorized as experimental.

Pilot Design
- Identify expected outcomes, success measures and Go/No-Go criteria for advancing to Expansion Stage (Referencing strategic priorities and past learnings). Plan pilot and data collection such that major risks or questions about the technology's scalability can be answered in a data-driven fashion, building from outcomes of technology proof of concepts and previous work.
- Ensure realistic timelines are developed to allow for activities that may require additional lead times (regulatory engagement, procurement and distribution of the PT, training, development of tech support strategy, etc.)

Patient Technology Implementation Framework
Technology Pilot

Approach (Continued)

Pilot Design (Continued)

- ➢ Identify and incorporate metrics that speak to the desired outcomes, success measures, and strategic priorities. Incorporate mechanisms for gathering stakeholder, patient, and other user feedback.
- ➢ Consider regulatory implications of the PT and, if appropriate, engage relevant health authorities for feedback. Considerations that may impact regulatory strategy include:
  - ▪ What is the original intended use of the PT? Does the use case differ from this intended use?
  - ▪ Does the PT have formal regulatory classification (i.e., is it a medical device)?
  - ▪ Will the PT be used solely in a clinical trial setting, or also in the commercial space?
  - ▪ Is the PT specific to a project or is it intended to be used across a platform?
- ➢ If possible, design control mechanisms or baseline measurements for your PT pilot, so that the impact of the PT on study conduct or outcomes can be quantified (e.g., control for learning effect, data access effects, etc.)

Pilot Conduct

- ➢ Conduct pilot and collect data on PT use
- ➢ Collect feedback on PT usability and implementation from sites, patients, and other stakeholders

Pilot Retrospective

- ➢ Analyze data and compare outcomes to success criteria
- ➢ Document and share learnings, both positive and negative. Provide feedback or insights that may impact strategy or future work, inclusive of patient feedback as appropriate.

Refine, Pivot, or Proceed:

Based on what was learned during the pilot, assess the technology's or use case's feasibility, value, and alignment with strategy. Make go/no-go decisions, pivots, or adjustments as appropriate. After each decision, refine the PT Experimentation Plan with new data, metrics, questions or hypotheses. Conduct additional Proof of Concept testing or re-visit technology options if necessary. If applicable, develop and refine business case, roadmap or risk analysis for additional pilots or for expansion.

Patient Technology Implementation Framework
Expansion

Stage Definition

Develop and implement a strategy for the scaled implementation of PT across studies, programs, therapeutic areas, and geographies, based on learnings from each of the previous stages

Approach

Once the PT has been deemed ready for mainstream use, develop a strategy to implement it more broadly. Construct this strategy based on learnings from pilots, including the following considerations:

Identify target use cases and populations
- Determine the desired scope for the use of PT at scale. Some PT may be appropriate for use with all patients, while others may only be appropriate or effective for certain sub-populations.
- Assess the level of difficulty with which the PT can be implemented across different geographies, therapeutic areas, or populations. This could be based on the regulatory environment, language, population acceptance of technology, or other factors.
- Prioritize potential use cases, geographies, therapeutic areas, or populations, and develop plan for scaling to them over time.

Develop business case and/or value proposition
- Identify specific factors or measures that speak to the value of the PT. Provide guidance on how to monitor these factors across studies, and to assess the value of the PT over time.
- If necessary or possible, estimate scale required to generate the desired returns outlined in the PT strategy.

Address potential issues identified during technology proof of concepts or pilots
- Ensure the PT and its use case(s) are feasible for all stakeholders. If the pilot produced findings around patient, site, or other stakeholder burden, take steps to address them.
- If additional technical capabilities, infrastructure, data streams or features are required for scaled implementation, create a plan to develop them.
- Re-evaluate previously identified risks. Ensure they have been mitigated, or that mitigation plans are in place.

Consider and document factors influencing future study planning
- Understand impacts on budget and timeline: Based on previous work and ongoing engagement with providers, model or estimate the impacts of the PT on study start-up costs, lead time, and overall study timeline. Consider potential economies of scale, and identify up-front costs associated with growing the PT and infrastructure.
- Understand regional or geographical considerations: Factors associated with scaled implementation may include regional variation in regulations, translation requirements, local characteristics of infrastructure or supply chain, and cultural differences impacting PT use.
- Solidify data management, storage, and reporting plans at scale: Ensure vendors and systems have all necessary capabilities to support the particular study, and ensure internal teams and systems have all necessary capabilities to manage this data.

Patient Technology Implementation Framework

Tools & Resources

Resource	Description
Accelerating Adoption of Patient-Facing Technologies in Clinical Trials: A Pharmaceutical Industry Perspective on Opportunities and Challenges *PT Workstream White Paper*	Building on a series of surveys and interviews with almost 600 subject matter experts, this paper explores the perceived opportunities and challenges the pharmaceutical industry faces when adopting patient-facing technologies. The authors identify trends across the industry and provides recommendations for addressing those challenges.
Patient-Focused Drug Development: Collecting Comprehensive Input *Draft Guidance for Industry, FDA*	This guidance is the first in a series of four methodological patient-focused drug development guidance documents developed by the FDA. This document (currently in draft) outlines the FDA's current thinking about methods that could be used during early-stage efforts to gain patients' perspectives on the clinical context.
Patient Priorities for Research *James Lind Alliance*	The James Lind Alliance sponsors 'Priority Setting Partnerships' of clinicians, patients, and caregivers in various therapeutic areas to identify and publish priorities for research and innovation in healthcare.
Mobile Technologies Recommendations *Clinical Trials Transformation Initiative*	The Clinical Trials Transformation Initiative (CTTI) published a series of recommendations for ➥ Mobile technology selection, ➥ Data collection, analysis, and interpretation, ➥ Data management, ➥ Protocol design & execution, and ➥ FDA submission and inspection when implementing mobile technologies in clinical trials.
Decentralized Clinical Trials Recommendations *Clinical Trials Transformation Initiative*	CTTI published recommendations and discussion on the legal, regulatory, and practical considerations affecting the use of mobile technologies in decentralized clinical trials.
Novel Endpoints Recommendations *Clinical Trials Transformation Initiative*	This framework and supporting materials outlines the steps recommended by CTTI for developing a novel endpoint measured by mobile technology for use in clinical trials.
Patient Technology Workstream Asset Page	This webpage is the primary location for solutions developed by the TransCelerate Patient Technology Workstream.