Continued Access to Investigational Products:

Guiding Equitable and Fair Decisions

MULTI-REGIONAL CLINICAL TRIALS

THE MRCT CENTER OF BRIGHAM AND WOMEN'S HOSPITAL and HARVARD

September 12, 2025

Learn more at www.mrctcenter.org

MRCT Center Disclaimer

- The opinions contained are those of the authors and presenters and are not intended to represent the position of Brigham and Women's Hospital or Harvard University.

- The MRCT Center is supported by voluntary contributions from foundations, corporations, international organizations, academic institutions, and government entities (see www.MRCTCenter.org), as well as by grants.

- We are committed to autonomy in our research and to transparency in our relationships. The MRCT Center—and its directors—retain responsibility and final control of the content of any products, results, and deliverables.

Speaker Disclaimer

- Ben Rotz is an employee at Eli Lilly & Company.
- Karla Childers is an employee at Johnson & Johnson.
- The opinions contained are those of the authors and presenters and are not intended to represent the position of Eli Lilly & Company and/or Johnson & Johnson.

Welcome!

Thank you for joining this webinar today!

Tips for today's session

- Use the Q&A for your questions – we will do our best to answer live.
- Feel free to use the Closed Captioning available on the Zoom toolbar.
- Most of the links in our presentations will be shared in the Chat.

The recording, slides, and any additional materials will be available next week.

The MRCT Center

The MRCT Center is a research and policy center focused on addressing the conduct, oversight, ethics, and regulatory environment of clinical trials.

Our Vision

Improve the integrity, safety, and rigor of global clinical trials.

Our Mission

Engage diverse stakeholders to define emerging issues in global clinical trials and to create and implement ethical, actionable, and practical solutions.

www.mrctcenter.org

Meet the Speakers

Karla G. Childers, BA, MSJ, MSBE
Vice President, Bioethics, Policy & Partnerships

Office of the Chief Medical Officer

Johnson & Johnson

Brandy Ellis,
Lived Experience Advocate

Ben Rotz, RPh
Associate Vice President, Global Medical Policy, Strategy, and Operations

Eli Lilly & Company

Moderated by:

Sarah White, MPH
Executive Director

MRCT Center

Today's Webinar

- Welcome and Overview of the MRCT Center
- Understanding why this is important….a case study.
- Project Background and available resources
- Revisiting the Case Study
- Audience Q & A

Case Study

Imagine…

You are leading the team responsible for planning the potential provision of post-trial continued access for an investigational product to treat a common – but difficult to treat - gastrointestinal condition in a phase 3 trial. There are a few therapies on the market globally but patient response to treatment varies and once a patient is stable on treatment, standard practice is to maintain that therapy until the patient progresses. Symptoms are not life threatening but can be difficult to manage and impact an individual's quality of life, including affecting their ability to work or care for others.

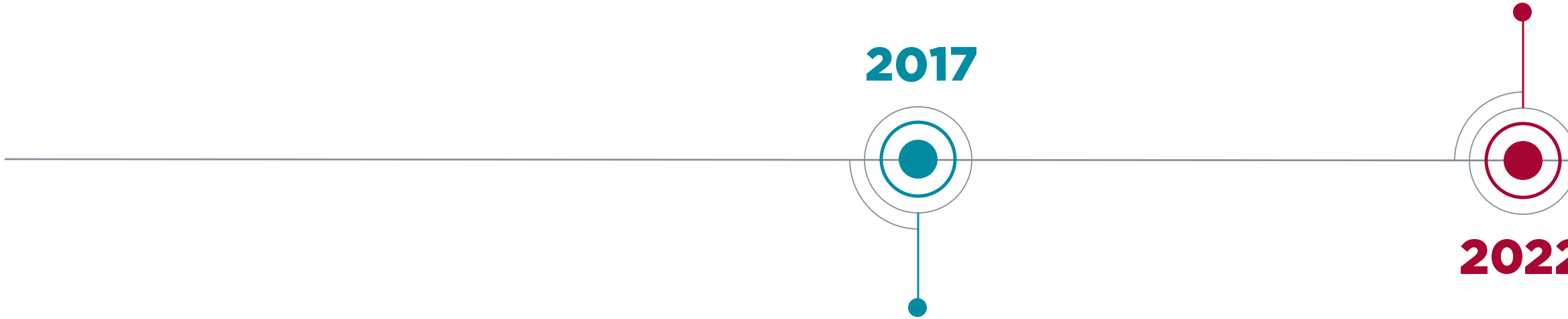
- At the start of the trial, your team reviewed your company's internal policies and conducted a landscape analysis of alternative therapies, concluding that post-trial continued access should be provided if there is benefit
  - The mechanism to provide the investigational product may be an extension trial, but depending on local regulations, this may differ across the sites
- Near the end of the trial when your team is doing the first primary readout, you see that the primary endpoint is not met *but* there is a subpopulation responding positively…

Continued Access

As defined by the Post-Trial Responsibilities: Continued Access Task Force

*The **continued provision** of the investigational medicine or **continued maintenance** of the investigational significant risk (SR) device for any clinical trial participant after participation in the trial. Continued access applies to medicines that are drugs or biologics and excludes vaccines. Some investigational interventions may require **unique supportive care** that should be considered by the sponsor, researcher, healthcare systems, or host country governments. Post-trial, continued access is a **shared responsibility** and should be determined before the trial begins.*

Our Work in Post Trial Responsibilities



2017

MRCT Center publishes the **Post-Trial Responsibilities: Continued Access to Investigational Medicines Principles, Guidance Document and Toolkit**

MRCT Center initiat
force" to update an
original resources

2022

Our Work in Post Trial Responsibilities

2017

MRCT Center publishes the **Post-Trial Responsibilities: Continued Access to Investigational Medicines Principles, Guidance Document and Toolkit**

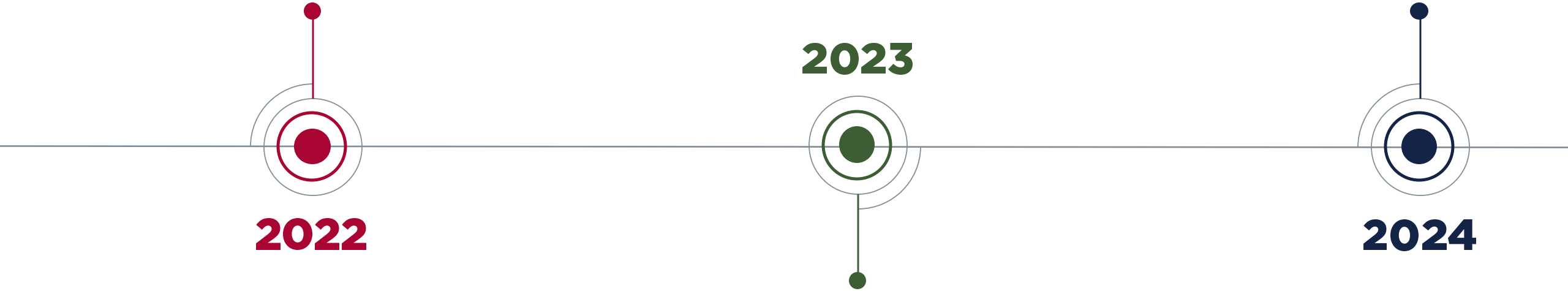
2022

MRCT Center initiates the "**PTR task force**" to update and add to the original resources

2023

The PTR task force develops **a revis of principles** with associated analys a **framework of responsibilities** for continued access to investigational medicines

Our Work in Post Trial Responsibilities



2022

MRCT Center initiates the "**PTR task force**" to update and add to the original resources

2023

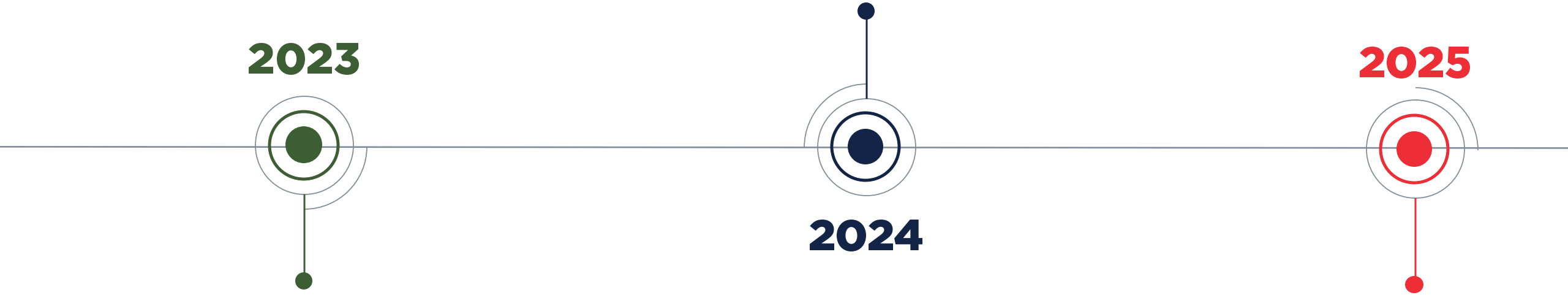
The PTR task force develops **a revised set of principles** with associated analysis and a **framework of responsibilities** for continued access to investigational medicines

2024

At the beginning of 2024, the
the **challenges of continued r**
investigational significant-risk
after a clinical trial

Trial
ess to
bles,

Our Work in Post Trial Responsibilities



2023

The PTR task force develops **a revised set of principles** with associated analysis and a **framework of responsibilities** for continued access to investigational medicines

2024

At the beginning of 2024, the work pivots to the **challenges of continued maintenance of investigational significant-risk (SR) devices after a clinical trial**

2025

The MRCT Center will focus on the **challenges of continued access in and middle-income countries**

Our Work in Post Trial Responsibilities

t the beginning of 2024, the work pivots to
he **challenges of continued maintenance of**
vestigational significant-risk (SR) devices
fter a clinical trial

The MRCT Center will focus on the
challenges of continued access in lower- and middle-income countries

MRCT Center's Post-trial Continued Access Taskforce

Task Force:

Karla Childers, *Co-Lead*, Johnson & Johnson
Dave Borasky, WCG IRB
Sean Daly, Takeda
Anthony Edmonds, Takeda
Brandy Ellis, Participant/Patient Advocate
Christine Grady, NIH Clinical Trials Center
Erika Hamilton, Sarah Cannon Research Institute, ASCO
Donald Harvey, Emory University School of Medicine, ASCO
Helen Kellar-Wood, Bristol Myers Squibb (BMS)
Diana Pankevich, Pfizer
Teri Pollastro, Participant/Patient Advocate
Anna Kang, Roche-Genentech
Benjamin Rotz, Eli Lilly and Company
Marjolein Willemen, Novartis
Crispin Woolston, Sanofi

Device Experts:

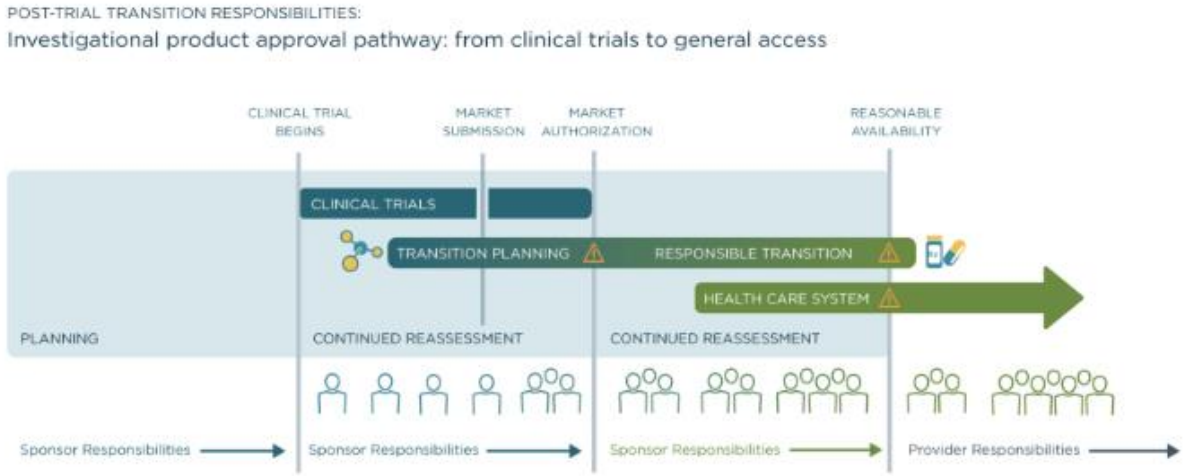
Joy Domingo, Johnson & Johnson
Elazer Edelman, MIT, BWH & Harvard Medical School
Saskia Hendriks, NIH
Richard Kuntz, MD. Formerly Medtronic
Gabe Lázaro-Muñoz, Harvard Medical School
Bruce Rosengard, Johnson & Johnson
Paul Underwood, Cardio MedSci.

MRCT Center:

Sarah White
Barbara Bierer
Kayleigh To

CLINICAL TRIALS & RESEARCH

Post Trial Responsibilities: Continued Access



VIEW LARGER

https://mrctcenter.org/project/post-trial-responsibilities/

What is Continued Access?

POST-TRIAL TRANSITION RESPONSIBILITIES:

Investigational product approval pathway: from clinical trials to general access

Sponsor Responsibilities

- Weigh interdependent criteria to inform decisions about the provision of continued access.
- Ensure that the given country agrees with the plan (duration and mechanism) for providing continued access to the investigational product.
- Ensure the criteria are equitably implemented and transparently communicated.

Sponsor Responsibilities

- Provide Access to Investigational Product (IP) that meet organization policy and decisions.
- Ensure access to medical care and infrastructure associated with delivery of IP
- Ensure equitable rollout of continued access to all similarly situated participants
- Consider, plan, and negotiate proper arrangements and clearly defined roles and responsibilities for the duration of post-trial continued access and maintenance phase.

Investigator Responsibilities

- Evaluate, determine, and communicate (to the participant and sponsor) whether the individual's benefit/risk assessment warrants continued access to the intervention(s) received during the trial in accordance with research program.

Sponsor Responsibilities

- Sponsor works with investigator and site to ensure transition of participant relationship to alternative access to IP, medical care, and infrastructure

Investigator Responsibilities

- If the participant will not continue to receive access to the investigational product, transition participant to a healthcare provider

Provider Responsibilities

- General access to the drug and medical care from payors, providers, government, etc.
- If not reasonably available (for whatever reasons):
- Sponsors should consider if there are limited scenarios where stopping treatment would be serious or life-threatening to the patient and warrant providing continued access.

RESOURCE

Principles of Post Trial Responsibilities

MULTI-REGIONAL CLINICAL TRIALS
THE MRCT CENTER OF BRIGHAM AND WOMEN'S HOSPITAL and HARVARD

Principles of Post-Trial Continued Access to an Investigational Product

November 2024

The Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard (MRCT Center) Post-Trial Responsibilities: Continued Access to an Investigational Product outlines a principled and practical approach to identify the ethical responsibilities that can, in turn, guide action to provide continued access to an investigational product at the conclusion of a patient's participation in a clinical trial. The foundation of the MRCT Center's work is grounded in 12 principles. The principles, accompanied by an analysis, should be read as a whole.

In the context of the work the MRCT Center defines **continued access** as the continued provision of the investigational medicine[1] or continued maintenance of the significant risk (SR) implanted device for any clinical trial participant after participation in the trial. Some investigational interventions may require specific supportive care that the sponsor, researcher, healthcare systems, or host country governments should consider.

1. **Research participants deserve consideration of continued access to an investigational product to which they receive benefit.**

   Research participants are exposed to the risks and inconveniences inherent in clinical trials to potentially benefit themselves, future patients, the sponsor,[2] and society as scientific knowledge advances. Research participants deserve consideration of continued access based on the following three ethical principles: (1) Following the reasoning of justice as reciprocity, research participants deserve consideration of continued access to a beneficial investigational product in return for their contributions. (2) The ethical principle of non-maleficence supports continued access if withdrawing an investigational product or not offering maintenance of a device at the end of a trial would cause known participant harm or long-term complications. (3) While research does not guarantee benefit to a participant, the principle of beneficence supports the consideration of continued access if the participant is benefiting from the intervention.

   References:

RESOURCE

Responsibilities to Investigational Products Framework:

- Investigational Medicines
- Investigational Significant- Risk Devices

MULTI-REGIONAL CLINICAL TRIALS
THE MRCT CENTER OF BRIGHAM AND WOMEN'S HOSPITAL and HARVARD

Post-trial, Continued Access Responsibilities to Investigational Significant-Risk Device Framework: Scenarios that require further consideration

Background: In the context o
continued provision of the inv
investigational significant risk
the trial. This document uses t

- Is intended as an impla
or welfare of a subject;
- Is purported or repres
presents a potential fo
- Is for a use of substanti
disease, or otherwise
potential for serious ris
- Otherwise presents a
subject.[1]

Please defer to local regulato

Some investigational interven
and/or maintenance that the
governments should consider
researchers, healthcare syster
before the trial begins, and be

Challenge: Sponsors and Res
post-trial continued access, th
continued maintenance of an
needed to ensure the device

MULTI-REGIONAL CLINICAL TRIALS
THE MRCT CENTER OF BRIGHAM AND WOMEN'S HOSPITAL and HARVARD

Post-trial, Continued Access Responsibilities to Investigational Medicines Framework: Scenarios that require further consideration.

July 2025

Background: In the context of the work, the MRCT Center defines **continued access** as the continued provision of the investigational medicine or continued maintenance of the investigational significant risk (SR) implanted device for any clinical trial participant after participation in the trial. Continued access applies to medicines that are drugs or biologics and excludes vaccines. Some investigational interventions may require specific supportive care that the sponsor, researcher, healthcare systems, or host country governments should consider. Post-trial, continued access is a shared responsibility among sponsors, researchers, and host country governments and should be determined before the trial begins, and before any individual gives their informed consent.

Challenge: Sponsors and Researchers generally agree upon the criteria used to determine post-trial, continued access, the regulatory milestones, and the pathways used to provide continued access to an investigational medicine. The timing between a pivotal trial of an investigational product and its regulatory approval is variable, as is the timing of commercial milestones such as market availability and reimbursement. It is in these windows that decisions about the provision of continued access must be made. There are, however, complex decisions that require further analysis.

The goal of the Framework of Responsibility is to develop a list of considerations that organizations can utilize to make equitable and fair decisions related to continued access to an investigational product. This framework was designed for sponsors and researchers developing investigational products and can be utilized to develop policy or guidance. Please note, that a framework to address considerations that sponsors and researchers can utilize related to *investigational significant-risk implanted devices* can be found here. This framework was developed based on the foundational work[1,2] of the 2017 MRCT Center Post-Trial Responsibilities Workgroup and has been expanded to clarify the current challenges related to post-trial access.

Responsibilities to Investigational Products Framework:

- Inter-dependent criteria

The MRCT Center has defined a set of **interdependent criteria**[4] related to the study program that may lead to continued access. Criteria may include, but are not limited to:

- Impact of discontinuation: The disease or condition under study is serious or life-threatening, and the research participant could be adversely impacted if access to the product were discontinued.
- Medical need: The investigational product addresses an unmet medical need in that no suitable therapeutic alternatives are available.
- No Access/Not Accessible: A physician cannot yet prescribe the product for the condition being studied.
- Research viability: The provision of continued access to the investigational product will not affect the viability of the research or the ability to complete the trial or other trials.
- Benefit/risk assessment: A positive overall study population benefit/risk assessment based on data analysis from first interpretable results or full study results.

Back to the Case Study

What do we do now?

- Were there safety issues or was it just a lack of efficacy?
- What do the safety and efficacy profiles look like for the treatment alternatives?
- Do we have any sense of how long these patients might be stable before needing to be switched again?
- Were there inclusion/exclusion criteria in the trial related to prior treatment that might affect what is now available to patients?
- Do we know what treatments are available in the various regions?
- What's the drug supply status?

Time for Q&A

Register for AI Digital Twins and Synthetic Data Webinar

Register for our Annual Symposium

Thank You!