



# MULTI-REGIONAL CLINICAL TRIALS

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

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**of Brigham and Women's Hospital and Harvard**  
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& VIRTUAL MEETING

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## Executive Summary

The [Multi-Regional Clinical Trials Center](#) of Brigham and Women's Hospital and Harvard (MRCT Center) is a research and policy center created to address the conduct, oversight, ethics, and regulatory environment of clinical trials, with a focus on multi-national clinical research. To do the work, we function as an independent convener to engage diverse stakeholders from industry, CROs, academia, patients and patient advocacy groups, non-profit organizations, and global regulatory agencies. Since 2009, the MRCT Center's efforts have resulted in the implementation of best practices, greater transparency, and improved safety for research participants. The MRCT Center's 2023 hybrid Annual Meeting was held on December 12<sup>th</sup> and 13<sup>th</sup> in Boston and Cambridge, MA. Attendees engaged in discussions of emerging issues facing global clinical trials, including ongoing work of the MRCT Center. New to this year's Annual Meeting, attendees engaged in sessions of the Research, Development, and Regulatory Roundtable (R3) and the MRCT Center's Bioethics Collaborative. The Annual meeting included:

**The keynote by Dr. Peter Arlett, Head of Data Analytics and Methods Task Force, European Medicines Agency (EMA)** discussed his mandate at the European Medicines Agency (EMA) which includes oversight of EMA's recently implemented Clinical Trials Regulation (CTR) and the European Union (EU) initiative Accelerating Clinical Trials in the EU (ACT EU). Dr. Arlett discussed the highlights and challenges coincident with the implementation of the CTR, ongoing implementation of the ACT EU, and a vision of his and EMA's work for 2024 and beyond. Discussion questions included inquiries about expectations for participant diversity, the 2025 deadline for the EU CTR, consent that allows for a broad future use of collected data, and challenges due to the EMA's authorization for the entire European Union.

### **Panel 1: Post-Trial Responsibilities: Continued Access to Investigational Products**

Post-trial, continued access to an investigational product is the continuity of that product after trial completion. This panel debated some of the complex issues that sponsors, investigators, and patients face with continued access to investigational medicines. Sarah White moderated the panel of experts that included: Karla Childers from Johnson & Johnson (Co-lead of this MRCT Center Project), Alta Charo from the University of Wisconsin-Madison, Sabrina Paganoni from Massachusetts General Hospital, and Sneha Dave from Generation Patient.

### **Panel 2: International Framework for Specimen Sharing – the Seattle Principles**

Mark Barnes from Ropes & Gray, Annette Schmid from Takeda, Marianne Bledsoe who is an independent consultant, Rita Lawlor from the University of Verona, and others have co-authored a document defining a set of international ethical principles for secondary research use of human specimens, known as "The Seattle Principles." This panel served to solicit input from meeting participants on several of the principles relating to returning research results,

safeguarding the welfare of specific communities, respect for consent of donors, and respecting scope of consent.

**Select MRCT Center Initiatives and ongoing Work** were presented by Dr. Barbara Bierer and Ms. Sarah White

### **Panel 3: Diversity Action Plans, Global Considerations**

In a compelling discussion moderated by Dr. Barbara Bierer, the imperative for clinical trials, in both the U.S. and in countries outside the U.S., to represent the populations who will benefit from medical advancements was the topic of the third panel. Lola Fashoyin-Aje from the FDA, Stacey Bledsoe from Gilead, and Cherie Butts from Biogen brought their insights, experiences, and challenges to the forefront, illuminating the ongoing paradigm shift in the industry.

The **Research, Development, and Regulatory Roundtable (R3)** included two sessions:

#### **Update on the European Union (EU) General Data Protection Regulation (GDPR), including a discussion of the EU-U.S. Data Privacy Framework**

The biggest change over the past year in GDPR regulation was the introduction of the EU-U.S. Data Privacy Framework (“EU-U.S. DPF” or “Framework”). Organizations participating in the EU-U.S. DPF can rely on it as a valid mechanism for the transfer of personal data from the EU to the U.S. There are concerns that the EU-U.S. DPF will face litigation. Panelists suggested that eligible companies may want to wait for any such litigation to be settled before joining the Framework. The Framework will benefit all organizations regardless of eligibility by providing a general stabilizing effect as well as a model for how GDPR-compliant data transfers can be conducted.

The panelists also discussed ongoing issues related to legal bases for processing and secondary research. If the data being processed include “special categories” of data,<sup>1</sup> as is often the case with clinical trial data, an Article 9 condition must also be satisfied. This becomes complex and time-consuming in multinational trials, as EU Member States have different positions regarding which basis for processing should be used. The panelists noted that the issues arising from the heterogeneity have gotten worse over the past year. If the original basis for data processing no

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<sup>1</sup> Article 9(1) GDPR. “Special categories” of personal data include “*personal data revealing racial or ethnic origin, political opinions, religious or philosophical beliefs, or trade union membership, and the processing of genetic data, biometric data for the purpose of uniquely identifying a natural person, data concerning health or data concerning a natural person’s sex life or sexual orientation.*”

longer applies, a new legal basis must be found for secondary processing. EU Member States differ on what they consider to be an appropriate basis for secondary processing.

The panelists also discussed challenges arising in decentralized clinical trials (“DCTs”). One of the main challenges with DCTs is that data are frequently collected by third-party vendors. These vendors are often startup companies that do not focus on health data and, therefore, frequently do not understand the risks involved with handling these data. It is critical for sponsors to vet third-party vendors and ensure that personal data will be handled properly before partnering with them. Finally, panelists discussed the increasing involvement of ethics committees in raising issues regarding GDPR compliance. One issue with this is that these committees often do not have privacy experts on them and, therefore, struggle to fully to understand the nuances of privacy regulations. A potential solution for this is a code of conduct.

### **Ensuring Integrity of Clinical Trial Data in a Decentralized Setting**

The collection and analysis of sound, reproducible data is fundamental to clinical research from both a scientific and an ethical perspective. Members of our expert panel discussed the data integrity challenges posed to clinical researchers by the combination of the advent of standalone for-profit research sites – whose profitmaking motivations may conflict with robust, deliberate research – and the recent rise in prevalence of decentralized clinical trials (DCTs) – where quality and oversight apparatuses have grown more distant from the collection of the data itself.

The panel discussion included descriptions of real-world instances of for-profit research sites lacking sufficient oversight and clinical quality assurance infrastructure that resulted in issues with study participant eligibility, breakdowns in study blinding, and substandard data collection. Limitations imposed by the COVID-19 pandemic resulted in many such sites remaining unmonitored and unaudited. The panel discussion then turned to the resources necessary to conduct robust DCTs. In particular, the panelists explained that DCTs are rarely, if ever, the least expensive research paradigm and stressed the need for striking a balance between recruiting hard-to-reach participants and maintaining data integrity. Following the formal panel discussion, a Q&A session was opened to guests in attendance. Panelists fielded questions on topics such as preventing research misconduct at for-profit sites, mitigating the risk of data loss in DCTs, and adapting research paradigms to accommodate more decentralized methodologies. Responses from our panelists tended to highlight the responsibilities incumbent upon sponsor organizations to establish and implement oversight and controls, regardless of variations in study design, and to prioritize Quality by Design when planning clinical studies over other considerations. Panelists did acknowledge opportunities for sponsor organizations to coordinate and simplify data collection and entry platforms to ease some of the workforce burden sites have experienced during the so-called “great resignation.”

**MRCT Center's Bioethics Collaborative** addressed the topic, "Gene Therapies: Probing the Ethics." This meeting enabled discussion of ethical issues associated with clinical trials of gene therapies (GTs). It served as an important grounding for the broader MRCT Center initiative on ethical, regulatory, and logistical challenges arising in the context of the clinical development of cell and gene therapies. Meeting attendees appreciated the number of unresolved and difficult issues in GT research. All members of the GT research community—including patients, patient advocates, and families—will need to work together to define and support best practices. Observation, reflection, and communication, as experienced at this meeting, will remain critical components of making that happen.

## Welcoming Remarks

Barbara Bierer, MRCT Center; Mark Barnes & Michael Beauvais, Ropes & Gray

Dr. Barbara Bierer, MRCT Center Faculty Director, welcomed in-person and virtual meeting participants. Dr. Bierer acknowledged and thanked the MRCT Center staff and many of the people attending the meeting, noting that the work could not have been done without them. She also thanked Ropes & Gray.

Mark Barnes, Partner at Ropes and Gray, and the MRCT Center Faculty Co-Director, introduced his colleague and friend, Michael Beauvais.

Michael Beauvais, Partner at Ropes & Gray, and the immediate past global co-chair of the life sciences and health care industry group at Ropes & Gray, welcomed participants to the Annual Meeting. He acknowledged the long history with the MRCT Center. Mr. Beauvais noted that the MRCT Center's mission and portfolio align with Ropes & Gray's mission and work, who provide services to many pharma clients.

## Keynote

Peter Arlett, Head of Data Analytics and Methods Task Force, European Medicines Agency (EMA)

Dr. Arlett joined the MRCT Center's Annual Meeting to discuss his mandate at the European Medicines Agency (EMA). Dr. Arlett's team is at the vanguard of EMA's efforts to transform clinical evidence. His team oversees the EMA's side effects reporting, real-world evidence (RWE), methodologies development, and clinical trials functions. In particular, Dr. Arlett oversees a team dedicated to running the operational and technology aspects of the EU's recently implemented Clinical Trials Regulation (CTR) with another team focused on the European Union (EU) transformation initiative Accelerating Clinical Trials in the EU (ACT EU). Dr. Arlett used his prepared remarks to discuss (i) some of the highlights and challenges coincident with the operationalization of the CTR, (ii) the transformation of clinical evidence in the EU, and (iii) a vision for EMA's work for 2024 and beyond.

### *Operationalization of CTR*

The EU updated its regulatory scheme regarding the conduct of clinical trials in response to a lack of increase in the initiation of new trials in the EU when compared to other regions, despite Europe historically having served as a driver of innovation in medicinal product development. Prior to the CTR, the EU treated regulation of clinical trials as a national competency, resulting in a fractured system of 27 unique sets of regulatory schemes to know and navigate for study sponsors, adding an immense administrative burden in addition to the high cost and high

failure rates that abound globally. Now that the CTR has gone live, study sponsors need only submit one application, as opposed to 27. The Clinical Trials Information System (CTIS) represents a comprehensive, end-to-end tool for submitting, processing, tracking, and making public all clinical trial applications with the EU. Likewise, ethics committees and national competent authorities access and review applications in the same shared space via CTIS. Moreover, CTIS also creates a public-facing register to permit the public to search for clinical trials, determine who principal investigators are, and identify study sites – theoretically empowering individuals interested in participating in a clinical trial to identify those opportunities.

A major operational challenge for the CTIS, in particular, and CTR, more generally, lies in the approximately 5,000 clinical trials that were already in process when CTR and CTIS went live, those planning to continue beyond January 2025 needing to transition to the new CTIS paradigm by that date. Even so, CTIS received nearly 3,000 clinical trial applications in 2023 and has already issued regulatory decisions on 1,800 of them.

#### *Transformation of Clinical Evidence and ACT EU*

Dr. Arlett introduced a multi-faceted approach to transforming clinical evidence by 2030. He noted the ample opportunity to leverage the availability of better healthcare data, advanced analytics (including Artificial Intelligence [AI]), and enhanced collaborations between international stakeholders to drive change. First, by improving transparency, the CTR can optimize use of time, effort, and money in clinical research by empowering researchers to learn from historical data and limit waste across the clinical research enterprise – waste of patients' investment, waste of time, and waste of money. Second, EMA has a vision to ensure the research question itself determines what types of evidence EMA will require to make its regulatory decisions. Clinical trial evidence will remain at the center of regulatory decision-making, but because EMA recognizes that clinical trials may not be able to answer every research question and because RWE has proven useful in some instances, EMA seeks to enhance its own regulatory flexibility to accept RWE (and other complementary forms of evidence) to drive regulatory decision-making where appropriate. Third, the EU has been successful at embedding patient perspectives at each step of the research flow. Lastly, committing to high levels of transparency underpins the public's trust; it also presents new challenges for study sponsors with respect to finding a balance between data transparency and proprietary information. Dr. Arlett's team has worked diligently in recent months to revise transparency rules to clarify the requirements for study sponsors, which should go live toward the middle of 2024.

EMA is also working to update and modernize clinical trials via the ACT EU initiative. Given the very unique sociopolitical structure of the EU, there has often been tension or resistance when seeking to make a disruptive change, but ACT EU represents the first time that the European Commission (the EU's legislative body), the EMA, and Member States have all come together in support of the same transformation initiative. ACT EU represents 11 concurrent workstreams



aimed at improving the efficiency of clinical trial operations and the decision-making and scientific advice functions of regulatory bodies in the EU.

### *2024 and Beyond at the EMA*

Even as EMA remains focused on the implementation and operationalization of CTR and CTIS, it focuses also on the future of regulatory medicine. In 2024, highlights include EMA hosting multistakeholder advisory boards on clinical trials and clinical trial analytics workshops and introducing new training resources and a regulatory helpdesk for non-commercial sponsors. A new pilot program to streamline regulatory (pre-clinical trial application) and scientific (design) advice is set to go live in the early 2nd quarter of 2024. EMA has also undertaken the development of a work plan regarding the use of AI in clinical research for 2024-2028. In addition to these internal efforts, the EMA remains focused on many international collaborations, including updates to the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) guidelines, the International Coalition of Medicines Regulatory Agencies, and a bilateral endeavor to modernize clinical trials regulations in conjunction with the FDA.

### **Discussion**

A meeting participant asked about expectations/requirements for participant diversity, specifically underserved, underrepresented, or hard-to-reach populations. Dr. Arlett clarified that concepts of inclusion and diversity can differ greatly around the globe. Historically, the EU has focused on inclusion and representation of women, children, and the differently abled under this umbrella while acknowledging that different global regulators likely require study designs to meet different benchmarks.

The discussion next turned to questions around the incorporation of pre-CTR/CTIS trials in the EU into CTIS by 2025. Members of the audience expressed concern about completing amendments in time and asked whether options existed for exempting studies from the transition if they are set to terminate within the first six months after the transition deadline. Dr. Arlett reaffirmed the January 2025 deadline and explained that CTR is very strict with respect to deadlines, and any delay would have to be authorized by the European Commission. Citing concerns about the learning process and the short 12-day response times for requests for information, some audience members found it impractical to provide adequate answers to the EMA and have instead decided to move trials to regions outside the EU. A participant then asked if changes driven by CTR have impacted the number of studies being conducted in the EU, noting challenges around deadlines and other issues (e.g., reporting breaches). Dr. Arlett noted no fall in the total number of CT applications in the EU. However, the overall impact of CTR-driven changes should include the size and impact of trials, and there will be regular reporting on impact going forward.

A question was asked about how the EMA and European Data Protection Board are thinking about clinical trials and GDPR requirements for research, for example, consent that allows for broad future use of data collected. Dr. Arlett shared that many in the EU are proud of the implemented data protections while acknowledging the complexities of different interpretations at the national level. He cited opportunities for industry to unite to develop good practice data protection codes of conduct as a powerful way to expedite receiving much-needed clarification from regulators. While EMA has done work on data protection as a technical advisor, data protection authorities have the mandate. Consequently, EMA's recommendations in this space are not binding because they are not the competent authority on data protection.

A meeting participant asked how the EMA is thinking about initiatives to bring evidentiary requirements or opportunities forward, which sparked a conversation about challenges due to the EMA's authorization for the entire European Union and Health Technology Assessment (HTA) agencies that are national or even sub-national. The new HTA legislation has a staggered implementation to create a structure for collaboration between regulators and HTA bodies and EMA is fully committed to its successful implementation.

Next, a question was posed about a requested code of conduct for pharmacovigilance and EMA's position on European Health Data Space (EHDS)'s opt-in/opt-out consent and opportunities to influence a law that has yet to be passed. Dr. Arlett urged perseverance and patience noting that the EHDS legislation will likely be adopted in 2024 and have a staggered implementation over many years. This was followed by a question regarding which specific documents submitted for Phase 1 adult trials and deferrals would be made public. Dr. Arlett remarked that only a short list of documents would be made public but urged the person to review public EMA guidance to confirm which specific documents those were. The final question asked about implementing improvements and efficiencies made during COVID in a post-COVID environment. While Dr. Arlett acknowledged derogations from protocols due to the pandemic, he expressed interest in future regulatory flexibility in public health emergencies and also reading across to more routine activities.

## Panel 1: Post-Trial Responsibilities: Continued Access to Investigational Products

Panelists: Karla Childers, Johnson & Johnson; R. Alta Charo, University of Wisconsin-Madison; Sneha Dave, Generation Patient; Sabrina Paganoni, Massachusetts General Hospital; Moderator: Sarah White, MRCT Center

**Sarah White, MPH: Executive Director at the MRCT Center** began the panel by introducing herself as the moderator and co-lead of the Post-Trial Responsibilities project. In 2017, the MRCT Center published the Post-Trial Responsibilities: Continued Access to Investigational Medicines [Principles](#), [Guidance Document](#), and [Toolkit](#) utilizing a case-based, principled approach produced by an international multi-stakeholder workgroup hosted by the MRCT Center. The goal was to evaluate and guide the ethical responsibilities of relevant stakeholders to provide continued access to investigational medicines after a patient's participation in a clinical trial. This guidance document and the frameworks it provides have been used by many of the organizations to guide policy and process related to this topic. After five years of use, the MRCT Center is updating this work under the guidance and direction of a small multi-stakeholder task force. The feedback gathered has alerted the Center of ongoing challenges in applying the ethical principles, policy implementation, and the unique challenges found in investigator-initiated trials, investigational devices, and continued access in under-resourced communities.

Ms. White provided some context of the project's scope before introducing the panel. Post-trial, continued access to an investigational product is the continuity of that product after trial completion. Post-trial, continued access is a shared responsibility of sponsors, researchers, and host country governments; there are multiple interdependent criteria used to decide whether to provide continued access to a study population and an individual.

The MRCT Center will release a revised set of principles with an associated analysis and a framework of responsibilities in early 2024. The purpose of the panel at the Annual Meeting was to discuss and debate some of the complex issues that sponsors, investigators, and patients face with continued access to investigational medicines.

**Karla Childers, BA, MSJ, MSBE: Johnson & Johnson**, co-lead of this project, offered additional opening remarks, including: the tension between individual vs. population-level benefit; the moral obligation that may depend on the rationale for stopping a research study; shared responsibilities amongst the different stakeholders; and the transition from the study period to product availability on the market.

Ms. White invited each panelist to provide opening thoughts on the biggest challenge of post-trial, continued access from their unique perspectives.

**Alta Charo, JD: Warren P. Knowles Professor Emerita of Law & Bioethics at the University of Wisconsin**, brings her bioethics perspective to this panel. She described the duality in how research participants are viewed: as vulnerable persons or individuals with an opportunity to receive a new medication. Second, she offered that although participants are in a research study, they may also be considered patients as they are receiving some form of healthcare. The fiduciary loyalty in a typical doctor-patient relationship may bleed into the investigator's relationship with the sponsor and research study. This role ambiguity may explain why some participants feel “betrayed” after a study if they believe they are left worse off by becoming dependent on a product they previously did not have access to, are no longer eligible to receive, or have become ineligible for future studies.

The intent of the MRCT Center's updated guidelines is to ensure that trial participants are not left worse off. This underlying principle should be clearly stated in the update, so that stakeholders can use this principle to derive policies that can assist them in working through challenging situations and provide practical advice.

Ms. Childers emphasized Prof. Charo’s position that the sponsor’s physician-investigators are noting the tension that arises from balancing their obligation to be good stewards of resources while caring for participants.

**Sabrina Paganoni, MD, PhD: Harvard Medical School/Spaulding Rehabilitation Hospital, Neurological Research Institute at the Massachusetts General Hospital and the Healey & AMG Center for ALS**, began with a brief introduction of the unique patient population she works with, individuals with ALS, a rapid and fatal disease with an average lifespan of three years. As a physician-scientist, Dr. Paganoni sees clinic patients once a week and otherwise focuses on research participants through an investigator-initiated group that works with industry in a unique collaboration. Dr. Paganoni has experience with open-label extension studies, expanded access to investigational products, and reimbursement through her current work environment. With few drugs on the market, the investigator-initiated group Dr. Paganoni is part of wants to change how studies are designed, engage with the community, and push toward active expansion and post-trial access.

**Sneha Dave: Generation Patient**, works in patient advocacy focused on adolescents and young adults with chronic or rare diseases in the non-profit she founded several years ago as a young adult herself with a chronic condition. When talking to members of her organization, Ms. Dave notes one question in particular arose, “Why would I participate in a trial if I can’t afford the therapeutic product afterward?” The young adult cohort is an age group that may be more likely to experience financial instability while navigating the complexities of newfound independence and autonomy. Additionally, Ms. Dave noted that there is often more centralized

and focused care while participating in a research study. Participants in a research study may receive additional support such as medical adherence and disease management. Once the trial is over the management and close oversight of the patient/participant's chronic condition may disappear. Getting the investigational product is important, but will there also be continued assistance in managing the disease?

Following introductions, Ms. White asked the panelists to share their thoughts on **evidence of benefit**. What happens if one participant receives benefit, but the group does not?

Dr. Paganoni stressed the differences in the Amyotrophic Lateral Sclerosis (ALS) population. As most patients have a life expectancy of three years and the randomized portion of a study could take 6-12 months, historically, ALS patients have only one chance of getting into a study. Further, participants may die even before the trial is completed. Through the community's advocacy work, ALS research studies are changing to offer an open-label study until the results are known.

Because of the state of the science, researchers often do not see the effects of the investigational product at the individual level as much as at the group level. The investigational products in ALS studies are not meant to cure but to attenuate the participant's decline. These endpoints generally require waiting until the end of the trial to evaluate, which is when the open-label study is bridging the gap. The community's advocacy work – challenging investigators to develop new statistical models to better utilize data – has also led to investigators to use data from the post-trial, open-label studies data as evidence, which has been used to approve recent drugs.

Prof. Charo wanted to delve deeper into the definition of “benefit.” The “easy” way to examine benefit is through objective measures, while the subjective measures and indirect benefits are more complicated to measure and translate. The subjective aspect of benefit needs to be part of the conversation and overall calculation by the sponsors and regulatory bodies when determining the benefit-risk ratio at both the population and individual levels. When the benefit is deemed substantial “enough,” whether subjectively or objectively, there should be accommodations, like continued access, at the end of the participant's part in a study.

Ms. White then pivoted the group to discuss moral obligation. Acknowledging research programs are stopped for various factors (e.g., safety, efficacy, futility, or a business decision), **how do the factors influencing the decision to stop an investigational product program affect the perception of moral obligation?**

Echoing Ms. White, Ms. Childers explained that there may be various reasons to stop a research study. For example, despite individual participants' perception of benefit, the sponsor may collect data that signals *a safety* concern, making it more straightforward to stop providing the investigational product because there is evidence to support the discontinuation. There is a

potentially increased obligation to halt access to an unsafe product. Another reason a sponsor may stop a study is a business decision. This could be something like deprioritizing a therapeutic area because of costs or competition entering the market. Ms. Childers indicated that from her perspective, such a discontinuation may lead to a heightened obligation to provide continued access until participants can be transitioned onto a different treatment.

Dr. Paganoni believes that as an investigator, her moral obligation is to develop drugs that meet local requirements to move research programs forward to benefit patients. For example, she was the Principal Investigator (PI) of a study that led to the approval of a drug for ALS. The study had ended, there were robust results, and an expanded access program was in place. The Food and Drug Administration (FDA) approved the drug in the US but the European Medicines Agency's (EMA) in the EU required results from a second trial for approval, leading to a global disparity in access to this drug.

Ms. White then turned to the foundational principle in the MRCT Center Post-Trial Responsibilities (PTR) guidance that **post-trial, continued access of an investigational product is a shared responsibility amongst all stakeholders**. At different points in the product development path, one stakeholder may have more responsibilities than others. The more the responsibility relates directly to the investigational product, the greater the responsibilities of the sponsor and investigator. The more the responsibility relates directly to medical care and access to infrastructure, the greater the responsibilities will transition to the government, payors, and healthcare providers. Ms. White challenged the panel to consider where the sponsor's boundary is, and if sponsors have a responsibility to engage with stakeholders at local levels to get approval of an investigational product and to ensure continued access.

Prof. Charo approached this question from an ethical rather than legal perspective. She does not believe sponsors have an infinite obligation to provide the investigational product. However, there should be a good faith attempt to anticipate the outcome, plan ahead, and conduct a responsible handoff so participants (now patients) can obtain essential care at the end of their trial participation and complete the study without feeling betrayed.

Ms. White acknowledged that patients often may not be concerned about *who* is responsible for providing access; they simply want access. The hypothetical question then posed was, what would patients want to know about these shared responsibilities?

Ms. Dave responded that this topic has brought up the idea of *potential alternatives*. If alternative therapies are available, why did the person decide to participate in a study in the first place? Specific to post-trial, continued access, Ms. Dave believes that sponsors cannot merely place a participant on an alternative therapeutic at the end of the study and expect all to be well. Remaining mindful of the cultural context of post-trial, continued access, Ms. Dave shared an example of a trial placed in India where participants did not want to be on the identified therapeutic medication even if it was working. They may want to get off the



investigational product as soon as possible. It is the responsibility of study teams to talk with participants to understand their wishes during and after a research study.

Another question raised **is the institution's role (e.g., academic medical centers)** in post-trial, continued access. The institution is often not a mentioned stakeholder, and Ms. White wanted to hear Dr. Paganoni's perspective on the institution's role in supporting investigators during the post-trial period.

In response, Dr. Paganoni clarified what a sponsor is. Often, it is thought of as a pharmaceutical company, but it could be the Investigator. For example, in some studies, Dr. Paganoni held the Investigational New Drug application (IND) from the FDA, so she was the regulatory sponsor, but not the financial sponsor. In those situations, she would work with companies who were the financial sponsors. This example demonstrates the shared responsibility as different stakeholders wear different hats depending on the trial, and the responsibility should be considered shared rather than one party vs. another.

Prof. Charo added that with post-trial, continued access, the participant reverts to being a patient in a more classic clinical situation. While the PI of the study may change positions or institutional/organizational affiliation, the former trial participant may remain at the original institution. In areas like gene therapy with requirements for years of follow-up, a physician may stop practicing, or the actual company may close. The bigger question to pose is how to structure post-trial, continued access, in a way that will allow for financing the follow-up in the long term.

## **Discussion**

An audience member from a pharmaceutical company raised their concern about using *life-threatening* as a criterion for post-trial, continued access, noting the sponsor may not be best positioned to decide what is *serious* because of the subjectivity of the lived experience of the condition in question. Many patients join studies because their current care is not helping them, and they need to try something new. To illustrate this point, an example from a migraine interventional study was shared. At the end of the study, there was no provision made for post-trial, continued access. One study participant was denied continued access to the investigational product that resulted in suicide because of the return of their migraines.

Ms. White acknowledged that the task force has also wrestled with this identified issue. Ms. Childers added that the assumption that every investigational product warrants some post-trial, continued access must be carefully evaluated, being mindful of the specific therapeutic area and whether other treatments are available and the likely risk of harm and its severity, as well as potential implications of removing a beneficial treatment. In the above example, the next step is to assess downstream impacts to determine what products are either not reimbursed or

may not be approved by a Health Technology Assessment (HTA) body. This is a downstream tradeoff that must be examined.

An audience member from another pharmaceutical company shared that their company weighs the regulatory definition of *serious* in their decision to offer post-trial, continued access. and conducts a landscape analysis to identify what suitable alternative treatments exist. For example, they may conduct research for Type 2 Diabetes interventions but will not commit at the beginning of the trial to offer post-trial, continued access because there so many alternatives are available.

Prof. Charo clarified her position, in response to a question from Dr. Bierer, about the potential justice implications for various scenarios of placing a trial where and when participants may not have access after the study. Teams should be aware of the location of trial placement; one should not place a trial in a location where the government or insurer is unlikely to cover the product even after approval. This could impact where sponsors decide to site their research studies. A further example was shared about a device being covered, but not the hospitalization necessary to implant the device. In such scenarios, only people with insurance will join the study, and those without insurance will not participate because they cannot afford the hospitalization. In such situations, one must ask *why* the studies would be placed there.

Finally, a question was raised regarding when participants may see a company's policy about post-trial, continued access. **Should the patient see the company's policy during consent to improve transparency and build trust?**

Ms. Childers responded that the company she works for generally does have a statement on their consent forms. Although the language appears, as with many aspects of informed consent forms, it is unclear if this is highlighted to potential participants or if potential participants understand the ramifications before they are part of a study.

The MRCT Center has also looked at consent forms and found that the post-trial, continued access language is often vague. As part of the PTR Task Force, one of the deliverables is to propose standard language that can be used in consent forms. Ms. White closed the panel by echoing Ms. Childers' position that participants eager to join a study may not be thinking about post-trial access. Questions remain about how to continue to communicate expectations with participants during a study.



## Panel 2: International Framework for Specimen Sharing – the Seattle Principles

Panelists: Marianna Bledsoe, Consultant; Rita Lawlor, University of Verona; Annette Schmid, Takeda; Moderator: Mark Barnes, MRCT Center

**Mark Barnes** introduced Marianna Bledsoe, Rita Lawlor, and Annette Schmid as panelists and introduced the [“Seattle Principles.”](#)

There are different international policies, procedures, and regulations that govern human research using human biospecimens and data, which has led to practical and administrative challenges for the conduct of collaborative research that involves human biospecimens and data. To address these challenges, a group with international representation is in the process of drafting and developing the “Seattle Principles” through ongoing engagement and collaboration. The intent is to articulate widely acceptable, practical, voluntary principles that can guide the curation, retention, and secondary use of human biospecimens and data. Development of these principles may promote global harmonization and standardization of expectations and behaviors related to the use and sharing of human biospecimens and associated data. Ultimately, if the principles lead to new ethical and practical norms, then this might lead to the adoption of new policies or legislation in different jurisdictions. Draft principles 2,3,5 and 9 were presented and reviewed with audience members for feedback and discussion.

### Discussion

**Annette Schmid** reviewed principle 9, Returning Research Results, which is of high interest to participants and relates to transparency. She opened the floor for discussion with questions about standards and expectations for the return of results and feasibility in the context of international data sharing. There was a robust discussion on the feasibility and logistical challenges related to returning results to the participants. Attendees acknowledged that over time the significance and relevance of results may change. Further, there may be practical and feasibility challenges as time progresses. Obligations to update returned genetic and genomic results in the light of new or emerging understanding of the significance are not clearly defined, even in the clinical or direct-to-consumer context, and may not be feasible. There was also concern about increased incentives to deidentify samples if there is a greater obligation to return results associated with identifiable biospecimens or data and potential implications on their value for future use. The importance of communicating return of results policies with specimen and data donors up front was highlighted. Dr. Bierer suggested that the principles may need to be iterated and updated periodically to keep up with technological changes and/or the evolving ethical and regulatory environment. Mr. Barnes indicated that the final version of the Seattle principle on return of results would need to appropriately reflect the temporal

dimensions, as he agreed that the obligation to return results would, for many reasons, decline over time from the study's conclusion. He also stressed that the most important point of the principle was to encourage researchers to think about what results they will generate and how they will approach the return of any clinically relevant findings.

**Marianne Bledsoe** shared the draft text for Principle 5, safeguarding the welfare of specific communities. She raised questions about expectations for community consultation, definitions of communities, and identification of appropriate representation. Some concerns were expressed regarding differentiating different types of communities. In the US, Native Tribes have sovereignty, a legal basis for consent, and rights. Outside of this context, it is not clear that ill-defined communities should be able to infringe upon an individual's decisions about participation in research. Annette Schmid stressed that the intent of the principle is not to take away the donor's autonomy but to speak to communities who might be involved with or impacted by the research. Several attendees agreed and stated that any time research is directed toward a specific community, it is important to engage with that community.

**Rita Lawlor** read the text for Principle 2, Respect for Consent of Donors, and Principle 3, Respecting Scope of Consent. She asked questions about the pros and cons of consents that are broad in scope and/or refer to unspecified future uses. Dr. Bierer and Mr. Barnes discussed consent practices for secondary research uses of excess biospecimens and associated data collected during the course of clinical care. Often, such research is minimal risk and granted a waiver of consent. In Europe, such biobanks have governance policies to which researchers must abide; however, biobank policies may need to be reviewed in light of the evolving landscape of GDPR.

Mr. Barnes concluded the panel discussion by thanking the panelists and attendees. Additional feedback and comments on the principles can be sent via email to [Seattleprinciples@ropesgray.com](mailto:Seattleprinciples@ropesgray.com).

## Select MRCT Center Initiatives and ongoing Work

Barbara Bierer & Sarah White, MRCT Center

Dr. Barbara Bierer and Ms. Sarah White provided an overview of selected MRCT Center initiatives and ongoing work.

**Decentralized Clinical Trials (DCT):** This is an ongoing project, in collaboration with Medable and a workgroup that has been addressing ethical issues in DCTs, through the lens of a participant journey through clinical trials. This group developed a list of considerations for Institutional Review Boards or ethics committees, which can be downloaded as a comprehensive [PDF](#) or as a simple [checklist](#). In 2024, the group will turn their attention to

issues such as Principal Investigator (PI) oversight and visibility, and the relationships with sponsor, sub- or site-investigator, health care provider, etc.

**Promoting Global Clinical Research in Children:** The MRCT Center developed a [toolkit for including young people in research](#). This includes principles and tools to implement the principles, such as recommendations on how to include young people as partners in research, and tools to make sure the voice of the child or young person is included in product development. Deliverables to date will soon be posted on a designated webpage. Many additional tools are under development, including participant-friendly materials for various age groups.

- **Health Technology Assessments (HTA)** in Europe have not focused on children. The MRCT Center organized a 2-day conference at the Brocher Foundation, Switzerland, early in 2023 with a focus on HTAs and pediatric populations.

**Health Literacy in Clinical Research:** The MRCT Center's long-term commitment to health literacy includes a [Clinical Research Glossary](#) that was piloted in 2020 and has been greatly expanded through co-creation both with a multi-stakeholder team to define additional words (to be released in March 2024) and with graphic design students to create unique images for each glossary term. In addition, the MRCT Center is collaborating with the Clinical Data Interchange Standards Consortium (CDISC) with a commitment to make the terminology of the MRCT Center Glossary part of the CDISC's global standard of definitions.

**Rapid Crisis Response Framework:** The MRCT Center will begin scoping a new project on rapid crisis response, starting with conversations with subject matter experts in early 2024. This work was borne out of the COVID-19 pandemic, natural disasters, and wars. The MRCT Center plans to develop a framework that addresses ethical responsibilities, regulatory flexibilities, clinical trial infrastructure, preparedness, and stakeholder responsibilities in times that challenge usual processes.

**Global Capacity Building:** The MRCT Center's long-term commitment to capacity building includes virtual and on-demand training. The online, on-demand, 10-module training "[Interpretation and Application of ICH E6 \(R2\)](#)", launched in February 2020, has been taken by thousands of people around the world. In addition, the MRCT Center has a "[IRB Health Literacy Training](#)" on-demand.

Two efforts were highlighted in greater depth:

- (1) The MRCT Center is developing an online, on-demand training course on Fundamentals of Research Ethics, which will be freely available on the OpenWHO platform in collaboration with the WHO. These modules are designed to help ethics committees understand areas to consider for interventional research in low- and middle-income countries. The first modules will be released in June 2024.

- (2) Systems optimization to advance ethics capacity across Africa and improve coordination between National Regulatory Authorities and Ethics Committees, in collaboration with the Bill and Melinda Gates Foundation and the WHO. Dr. Bierer has been traveling to Africa, co-developing a common [ethics oversight assessment tool](#) to assess and report gaps in their systems, in an effort to develop Centers of Excellence in the ethical review of research.

Ms. White shared that members of the MRCT Center Executive and Steering Committees met to discuss new geographic areas for clinical research since collaborative and harmonized efforts help build capacity for clinical research in new geographic regions. Some of the suggested actions were to develop a capacity heat map to identify and communicate potential challenges and opportunities and to develop a checklist to map and understand the capabilities when a sponsor engages with a new site.

**ICH Training:** The MRCT Center, as an ICH Training Associate, is developing a series of pre-recorded introductory and in-depth videos and modules to interpret and apply ICH Guidelines, including E6(R3), E8(R1), and E17. It will include experiences from low- and middle-income countries (LMICs). The MRCT Center is currently producing an introductory overview video about ICH E8(R1) and developing in-depth training modules on ICH E8(R1). Training modules on ICH E6(R3), and ICH E17, and finally an integration of how the three guidance documents should be used together, will follow in 2024 and 2025.

**Joint Task Force for Clinical Trial Competency (JTF):** One of the first projects of the MRCT Center was to develop a set of competencies for clinical research professionals. The resulting JTF Framework, first published in 2014, includes eight domains with competencies and skills that are needed to conduct a clinical study with integrity and quality. Over the intervening years, this framework has been organically translated into ten languages as requested by users, and the MRCT Center organizes biannual meetings with ~40 participants from around the world to discuss its implementation. Currently, the domain of Data Management and Informatics is being revised, and a new set of competencies to support patient partner participation in the research development process is being considered.

**Diversity, Equity, and Inclusion (DEI):** For the last eight years, the MRCT Center has been working on developing a comprehensive diversity and inclusion effort for clinical trials. In the last year, the MRCT Center expanded its DEI efforts to focus on involvement of people with disabilities. As part of the work on disability, the MRCT Center launched the [Accessibility by Design in Clinical Research Toolkit](#) in May 2023, and Dr. Willyanne DeCormier Plosky spoke on the topic at numerous fora. These included the November 2023 FDA/Clinical Trials Transformation Initiative (CTTI) Public Workshop to Increase Clinical Trial Diversity and the December 2023 PRIM&R Annual Meeting. Key points are that people with disabilities are the largest minority population in the U.S., many people with disabilities have intersecting identities as racial or gender minorities, and there is a movement to shift from exceptionalism

to inclusion in research. The MRCT Center also began a working group on LGBTQIA+ Inclusion by Design in Clinical Research, to support intentional representation of all communities, and we anticipate developing 6-8 tools on topics such as inclusive imagery and language, data collection, and accountability in mid-late 2024.

The MRCT Center is in the process of creating a Roadmap for Global Diversity Equity and Inclusion (GDEI) to provide guidance on how to consider data when combined in global trials. This work is spearheaded by Katharine Wright. The MRCT Center has also collaborated with CTTI, Faster Cures/Milken Institute, and the National Academies for Sciences, Engineering, Medicine (NASEM) on the Diversity Convergence Project to align organizational efforts and drive collective action for systems change in DEI. The four organizations meet every week and have had three conferences, with a fourth conference scheduled in 2024, and a plan to develop a product for Clinical Trials Day in May. There has also been much work to get a perspective on what DEI should look like in clinical trials to be compliant with regulations. A new collaborative involves the Association for Accreditation of Human Research Protection (AAHRPP), Public Responsibility in Medicine and Research (PRIM&R), Mass General Brigham (MGB) and the MRCT Center to collate and enhance DEI tools with the aim of increasing their visibility, uptake, and adoption.

Dr. Bierer thanked the MRCT Center team for all their efforts.

## Panel 3: Diversity Action Plans, Global Considerations

Panelists: Lola Fashoyin-Aje, U.S. Food and Drug Administration; Stacey Bledsoe, Gilead; Cherié Butts, Biogen; Moderator: Barbara Bierer, MRCT Center

Dr. Barbara Bierer introduced the panelists. Dr. Bierer credited the continual efforts of the panelists to convey that clinical trials should represent the populations who will use the tested product, importance of understanding safety and efficacy among different populations, and making all people feel included in the work. Dr. Fashoyin-Aje discussed the FDA perspective on diversity efforts for clinical trials (both in and outside the US) and what DEI topics the FDA feels are important to educate clinical trial stakeholders.

**Dr. Lola Fashoyin-Aje** highlighted the imperative to elevate the dialogue around Diversity, Equity, and Inclusion (DEI) beyond policy to concrete actions within the realm of clinical drug development. She emphasized the necessity to integrate DEI considerations into clinical trial operations seamlessly, moving beyond a simple commitment to a more nuanced operational level.

Dr. Fashoyin-Aje acknowledged that while larger pharmaceutical companies have dedicated teams to address DEI issues, the translation of intent into specific goals and objectives remains a formidable challenge. The key concern expressed was the failure to consistently incorporate DEI throughout the entire trial process, urging a reevaluation of practical and sustainable approaches.

**Ms. Stacey Bledsoe** shared insights into the challenges of building a DEI strategy. She described the process as "building the ship as we are flying it" and stressed the importance of laying a strong foundation, establishing Standard Operating Procedures (SOPs), and rethinking ways to incorporate epidemiological data beyond cancer.

**Dr. Cherie Butts**, drawing from experiences at Biogen and FDA, emphasized the need for diversity not only in the trial participants but also in preclinical packages. She highlighted the significance of a shift in mindset, noting that it should not require more financial resources but rather a change in approach, including how investigators are engaged.

Dr. Butts emphasized the transactional nature of many clinical trials and advocated for a more relational approach. She stressed the importance of establishing rapport, spending time listening to and understanding the burdens and sensitivities of key stakeholders in the clinical trials process. This includes trial coordinators and investigators. She challenged assumptions about minority participation and underscored the value of listening to patients to patient groups and understanding their perspectives. She shared an example of increasing enthusiasm for participation in systemic lupus erythematosus trials, where patients stated that having a healthy alternative to vending machine food would make a significant difference in their experience.

Ms. Bledsoe and Dr. Butts delved into the challenge of embedding DEI into the business aspects of clinical trials. Ms. Bledsoe highlighted the need to discuss diversity even in seemingly mundane documents like investigator brochures. Dr. Butts shared her thoughts, noting a goal of achieving a representative population instead of debating Diversity, Equity, Inclusion, Justice.

Dr. Bierer raised questions about the variation in approaches across therapeutic areas and why the focus often starts at phase 3. Dr. Fashoyin-Aje emphasized the importance of aligning goals with trial outcomes and the need for a mindset change in how clinical trials are perceived.

Ms. Bledsoe discussed the challenge of obtaining diversity data, particularly outside of the United States, and the need for an evolutionary approach. Dr. Fashoyin-Aje cautioned against overly focusing on race and emphasized the importance of data being generalizable to the US population and medical care settings. Ms. Bledsoe also touched on the apprehension within teams about setting ambitious diversity goals. She stressed the need to 'play within the sandbox' of achievable goals and to consider what happens if specific targets are not met.



## Discussion

A participant raised concerns about the heterogeneity in diversity plans and asked about the ideal time to introduce DEI considerations. Dr. Fashoyin-Aje recommended **integrating DEI considerations before writing the Target Product Profile (TPP)**, to help ensure that it aligns with the epidemiology of the disease.

Another participant inquired about the impact of the recent FDA workshop on participants' perspectives. Ms. Bledsoe expressed both fear and motivation, emphasizing the need for organizations to expand their definitions of diversity comprehensively.

An attendee suggested leveraging patient engagement groups as a starting point for early consideration of DEI. Dr. Butts highlighted the importance of examining what others are doing (success stories), especially in the rare disease space.

In summary, the discussion underscored the critical need to move beyond siloed discussions on DEI, urging the industry to bridge the gap between intent and action in clinical drug development. The participants emphasized the importance of an evolutionary approach, active listening, and the early integration of DEI considerations into the entire clinical development process. The overarching goal is to ensure that clinical trials are not just transactional but, more importantly, relational experiences that reflect the diverse and representative population they aim to serve.

## Closing Remarks

Barbara Bierer & Sarah White, MRCT Center

Dr. Bierer was enthusiastic that the day's discussions were helpful to advance the MRCT Center's thinking. In response to an earlier question, Dr. Bierer explained that topics discussed during the Annual Meeting are related to ongoing projects of the MRCT Center and welcomes feedback. She encouraged participants to send their comments.

Ms. White summarized the meeting with the word "wrestle." This word was heard across most of the panels. The MRCT Center is wrestling with difficult and challenging issues, such as EU legislation, ethical principles in post-trial responsibilities, Diversity Action Plans, etc.

Dr. Bierer expressed a sincere thank you to MRCT Center staff, Mark Barnes, Ropes & Gray, speakers of today's meeting, and MRCT Center Executive Director, who, in turn, thanked the MRCT Center Faculty Director.

## December 13, 2023, Research, Development, and Regulatory Roundtable (R3)

For meeting summary, R3 sponsors may contact [mrct@bwh.harvard.edu](mailto:mrct@bwh.harvard.edu).

## December 14, 2023, MRCT Center Bioethics Collaborative

For meeting summary, please click [here](#).

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## Appendix 1: Meeting Agenda

### MRCT Center 2023 Annual Meeting

**Wednesday, December 13, 2023**

Ropes & Gray, Prudential Tower, 49<sup>th</sup> floor,  
800 Boylston Street, Boston, MA 02199

Time	Topics/Speakers
8:00 – 8:15 AM	<b>Breakfast &amp; Registration</b>
8:15 – 8:30 AM	<b>Welcoming Remarks</b> <ul style="list-style-type: none"> <li>• Barbara Bierer, MRCT Center</li> <li>• Mark Barnes, Ropes &amp; Gray and MRCT Center</li> <li>• Michael Beauvais, Ropes &amp; Gray</li> </ul>
8:30 – 9:15 AM	<b>Keynote</b> <ul style="list-style-type: none"> <li>• Dr. Peter Arlett Head of Data Analytics and Methods Taskforce EMA (European Medicines Agency) Co-chair HMA-EMA Big Data Steering Group Honorary Professor, London School of Hygiene and Tropical Medicine</li> </ul> <b>Moderator:</b> Barbara Bierer, MRCT Center
9:15 – 10:15 AM	<b>Panel 1: Post-Trial Responsibilities: Continued Access to Investigational Products</b> <p><b>Panelists:</b></p> <ul style="list-style-type: none"> <li>• Karla Childers, Johnson &amp; Johnson</li> <li>• R. Alta Charo, University of Wisconsin-Madison</li> <li>• Sneha Dave, Generation Patient</li> <li>• Sabrina Paganoni, Massachusetts General Hospital</li> </ul> <b>Moderator:</b> Sarah White, MRCT Center
10:15 – 10:30 AM	<b>Break</b>
10:30 – 11:30 AM	<b>Panel 2: International Framework for Specimen Sharing – the Seattle Principles</b>

	<p><b>Panelists:</b></p> <ul style="list-style-type: none"> <li>• Marianna Bledsoe, independent Consultant</li> <li>• Rita Lawlor, University of Verona</li> <li>• Annette Schmid, Takeda</li> </ul> <p><b>Moderator:</b> Mark Barnes, Ropes &amp; Gray and MRCT Center</p>
11:30– 12:00 PM	<p><b>Select MRCT Center initiatives and ongoing work</b></p> <p><b>Presenters:</b></p> <ul style="list-style-type: none"> <li>• Barbara Bierer, MRCT Center</li> <li>• Sarah White, MRCT Center</li> </ul>
12:00 – 1:00 PM	<p><b>Lunch for all Attendees</b></p>
1:00 – 2:00 PM	<p><b>Panel 3: Diversity Action Plans, Global Considerations</b></p> <p><b>Panelists:</b></p> <ul style="list-style-type: none"> <li>• Lola Fashoyin-Aje, FDA</li> <li>• Cherié Butts, Biogen</li> <li>• Stacey Bledsoe, Gilead</li> </ul> <p><b>Moderator:</b> Barbara Bierer, MRCT Center</p>
2:00 – 2:15 PM	<p><b>Research, Development, &amp; Regulatory Roundtable (R3) Welcome</b></p> <ul style="list-style-type: none"> <li>• Mark Barnes, Ropes &amp; Gray and MRCT Center</li> </ul>
2:15 – 3:15 PM	<p><b>R3 Topic 1:</b> Update on the GDPR, including discussion of the EU/US data privacy framework</p> <p><b>Speakers:</b></p> <ul style="list-style-type: none"> <li>• Sara Berkson, Vertex</li> <li>• Hannah Bracken, U.S. Dept of Commerce</li> <li>• Jennifer Courture, Consultant</li> <li>• Vicky Perez Riu, Takeda</li> </ul> <p><b>Moderator:</b> David Peloquin, Ropes &amp; Gray</p>
3:15 – 3:30 PM	<p><b>Break</b></p>
3:30 – 4:30 PM	<p><b>R3 Topic 2:</b> Ensuring Integrity of Clinical Trial Data</p> <p><b>Speakers:</b></p>

	<ul style="list-style-type: none"> <li>• Andrew Lee, Merck</li> <li>• Nicholas Kenny, Syneos Health</li> <li>• Greg Licholai, ICON plc</li> </ul> <p><b>Moderator:</b> Mark Barnes, Ropes &amp; Gray and MRCT Center</p>
4:30 – 4:45 PM	<p><b>Wrap-up and closing</b></p> <ul style="list-style-type: none"> <li>• Sarah White, MRCT Center</li> <li>• Barbara Bierer, MRCT Center</li> </ul>
5:00 – 6:15 PM	<p><b>Cocktails and Appetizers</b></p>

## Appendix 2: Speaker Biographies

### MRCT Center Leadership

#### **Barbara Bierer, MD**



Barbara is the Faculty Director of the Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard (MRCT Center); Professor of Medicine, Harvard Medical School and Brigham and Women's Hospital, Boston; and a hematologist/oncologist. She is also the Director of the Regulatory Foundations, Ethics and Law Program of the Harvard Clinical and Translational Science Center and the Director of Regulatory Policy, SMART IRB. She is Faculty in the Center for Bioethics, Harvard Medical School, and Affiliate Faculty in the Petrie-Flom Center for Health Law Policy, Biotechnology, and Bioethics at

Harvard Law School. Previously she served as Senior Vice President, Research, at the Brigham and Women's Hospital for 11 years, and was the institutional official for human and animal research, for biosafety, and for research integrity. She initiated the Brigham Research Institute and the Innovation Hub (iHub), a focus for entrepreneurship and innovation. In addition, she was the Founding Director of the Center for Faculty Development and Diversity at the BWH.

In addition to her academic responsibilities, Dr. Bierer served or serves as Chair of the Secretary's Advisory Committee for Human Research Protections, Department of Health and Human Services (2008-2012); as a member of the National Academies of Sciences Committee on Science, Technology and the Law (2007-2016); on the Boards of Directors of Public Responsibility in Medicine and Research (PRIM&R; 2011-2020), Management Sciences for Health (MSH; 2013-2022), Vivli (2017-), Clinithink (2015-), and North Star Review Board (2020-). She chairs the Board of Trustees of the Edward P. Evans Foundation, a foundation supporting biomedical research. She has authored or co-authored over 260 publications.

**Sarah White, MPH**



Sarah is responsible for developing, defining, and implementing the overall strategy and vision for the Center as well as oversee all management aspects of the MRCT Center functions. Sarah has over 20 years of experience in human subjects' research including experience at both academic medical centers and industry.

Prior to joining the MRCT Center, Sarah was the Director of the Human Research Quality Improvement Program (QI Program) at Partners' Healthcare in Boston, Massachusetts. In this capacity, she was responsible for strategic planning and oversight of the QI Program activities across the human research communities at Partners Healthcare, including Massachusetts General Hospital and Brigham and Women's Hospital. In addition, Sarah oversaw FDA Sponsor-Investigator support and the centralized support of clinical trials registration and disclosure. Sarah is the co-chair of the national Clinical Trials Registration Taskforce, a large consortium of academic medical centers, hospitals and universities that identify best practices, develop tools, and serve as a communication forum associated with the requirements for clinical trials registration and results reporting that affect US academic health centers. Sarah received her undergraduate degree from Dartmouth College and her MPH from Boston University School of Public Health.



**Mark Barnes, JD**

Mark is the Faculty Co-Director of the MRCT Center, Partner at Ropes and Gray LLP, and Visiting Lecturer at Yale Law School. Mark's law practice and his teaching at Yale focus on healthcare law and finance, human and animal research, stem cell and genetic research, research grants and contracts, research misconduct, and international research. Mark formerly served at Harvard as the Senior Associate Provost and University Senior Research Officer and started and directed Harvard's HIV/AIDS treatment programs in Nigeria, Tanzania and Botswana. Mark has held senior appointed positions in the New York City and State departments of health. In 2019, he was named the "Legal Innovator of the Year" by the Financial Times, and over the past two decades has served in various capacities on the HHS Secretary's Advisory Committee on Human Research Protections and its subcommittees.

## Keynote Speaker



**Peter Richard Arlett, MD, FRCP, FPPM**

Peter Arlett is Head of the Data Analytics and Methods Taskforce at the European Medicines Agency. In this role he leads on operations and transformation on clinical evidence at the EMA including clinical trials, real world evidence, safety reporting and data science including AI. He is Chair of the EMA Data Board, Co-Chair of the HMA-EMA Big Data Steering Group, Co-chair of the EMA AI Coordination Group, Co-chair of the Vaccine Monitoring Platform Steering Group, and Member of the ACT EU Steering Group. Prior to taking up this role in 2020, he held leadership roles within the EMA in the areas of pharmacovigilance, epidemiology, and risk management.

Prior to starting at EMA in 2008, Peter worked on new legislation and international collaboration for the European Commission, was the UK delegate to the European Committee for Human Medicinal Products, and was an assessor and manager at the UK's MHRA. He has a medical degree from University College London and began his career as a hospital physician in Oxford and London.

In addition to his role at EMA, Peter is an Honorary Professor at the London School of Hygiene and Tropical Medicine. He is also a Fellow of the Royal College of Physicians of Edinburgh and of the Faculty of Pharmaceutical Medicines of London.

*Moderator: Barbara Bierer, MRCT Center*

## **Panel 1: Post-Trial Responsibilities: Continued Access to Investigational Products**



**Karla G. Childers, MSJ, MSBE**

Karla joined Johnson & Johnson (J&J) in 2013 in the Office of the Chief Medical Officer where she has been leading and coordinating bioethics-based, science and technology policy projects. Ms. Childers is the Chair of the J&J Bioethics Committee, which serves as an internal forum providing advice on bioethical questions. She serves as a bioethics subject matter expert for various internal and external policy work and coordinates the internal bioethics educational program sponsored by the Office of the Chief

Medical Officer. Ms. Childers began her career in industry 18 years ago as a bench chemist before moving into policy and ethics roles. She received her Bachelor of Arts in Chemistry from Indiana University-Purdue University in Indianapolis and a Master of Science in Jurisprudence with a concentration in Health Law from Seton Hall Law School. She is also a graduate of Columbia University with a Master of Science in Bioethics.



**R. Alta Charo, JD**

R. Alta Charo is the Warren P. Knowles Professor Emerita of Law & Bioethics at the University of Wisconsin, where she taught biotechnology regulatory policy, public health law and medical ethics for over 30 years. She has worked in government as a legal and policy analyst for the US Congress' former Office of Technology Assessment, the US Agency for International Development and the US Food & Drug Administration. In addition, she served as a member of the NIH Human Embryo Research Panel, the NIH working group on use of chimpanzees in research, and from 1996-2001 was a member of President Clinton's National Bioethics Advisory

Commission. Charo has testified before Congress on topics ranging from cloning to use of fetal tissue in research, and has been elected to membership in the US National Academy of Medicine -- where she co-chaired its seminal reports on embryonic stem cell research and on human genome editing -- as well as to the American Association for the Advancement of Science, the American Academy of Arts and Sciences, and the German Leopoldina. She helped draft the latest guidelines on stem cell research and therapy for the International Society for Stem Cell Research, and she served on the World Health Organization's expert advisory committee on human genome editing. Professor Charo now works as an independent bioethics consultant advising government, professional organizations and companies on ethical and regulatory issues relating to clinical trials of emerging therapies, ethical and political issues raised by reproductive technologies, and policy choices presented by bio-engineering animals for de-extinction and conservation purposes.





### **Sneha Dave**

Sneha graduated from Indiana University in May 2020 where she majored in chronic illness advocacy as well as journalism. She created [Generation Patient](#) to develop support systems for adolescents and young adults with chronic conditions across the U.S. and internationally. She is proud to work with a team composed entirely of young adults with chronic conditions and also to keep Generation Patient and CCYAN independent from the pharmaceutical and insurance industries. Sneha has completed an undergraduate research fellowship in health policy at Harvard T.H. Chan School of Public Health. Sneha has spoken on Capitol Hill, featured nationally on C-SPAN, and is a past contributor for U.S. News and World Report. She has served on the Democratic National Committee Disability Policy

Subcommittee and recently joined the Midwest Comparative Effectiveness Public Advisory Council, an independent appraisal committee of the Institute for Clinical and Economic Review. She also serves on the FDA Patient Engagement Collaborative, in a grantmaking committee with the Robert Wood Johnson Foundation, and as part of the advisory board for the Yale Collaboration Regulatory Rigor, Integrity, and Transparency. Sneha was awarded two academic fellowships with the Association of Health Care Journalists. For her work, she was selected as one of the most influential teenagers in 2018 by the We Are Family Foundation and was recognized as an American Association of People with Disabilities Emerging Leader in 2020.



### **Sabrina Paganoni, MD, PhD**

**Sabrina Paganoni** is an Associate Professor of PM&R at Harvard Medical School / Spaulding Rehabilitation Hospital. She is also the Co-Director of the Neurological Research Institute at the Massachusetts General Hospital and physician scientist at the Healey & AMG Center for ALS. Her research focuses on clinical trials and therapy development for ALS. She has served as PI of several ALS clinical trials and has been using novel trial designs, novel endpoints, and digital technology tools to innovate the way investigational products are tested in ALS. She is the co-PI of the HEALEY ALS Platform Trial, the first platform trial for ALS in the world. She recently reported the positive results of the CENTAUR trial and is the co-Chair of the global PHOENIX trial. Her research has been funded by the NIH, non-profits, and industry; she

published more than 100 peer-reviewed manuscripts and received several awards for her work including the 2021 Top 10 Clinical Research Achievement Award.

*Moderator: Sarah White, MRCT Center*



## Panel 2: International Framework for Specimen Sharing – The Seattle Principles



**Marianna J. Bledsoe, MA**

Marianna is an independent consultant on biobanking and research policy issues. She is also Editor-in-Chief for the journal, *Biopreservation and Biobanking*.

Involved in biobanking for more than 25 years, Marianna provided scientific direction and oversight for numerous biobanks at the US National Cancer Institute (NCI) and the Department of Veterans Affairs. As Deputy Associate Director of the Clinical Research Policy Analysis and Coordination Program in the Office of Science Policy, Office the Director, National Institutes of Health, she participated in the development of federal policies and educational documents related to biobanking, genomic data sharing, privacy and confidentiality, and health information technology. These included the US Department of Health and Human Services Secretary's Advisory Committee for Human Research Protections recommendations on specimen research, the Council of Europe Recommendations on Human Biological Materials(2006), the Organization for Economic Cooperation and Development Guidelines on Human Biobanks and Genetic Research Databases, the NCI Best Practices for Human Specimen Resources and the International Society for Biological and Environmental Repositories (ISBER) Best Practices for Repositories. More recently, Marianna has participated in the development of biobanking standards, including ISO 20387, Biotechnology — Biobanking — General requirements for biobanking. She has co-authored numerous publications related to biobanking, particularly on ELSI issues. Marianna was ISBER President from 2007 - 2008. She received the 2012 ISBER Distinguished Leadership and Service Award and 2019 ISBER Outstanding Achievement in Biobanking Award. Marianna received her Masters degree in organic Chemistry from Johns Hopkins University.



**Helen Morrin**

Helen is a distinguished professional in the field of cancer research. She serves as the curator of Otago University's He Taonga Tapu Cancer Society Tissue Bank and maintains an active role in the Mackenzie Cancer Research Group, with a specific interest in colorectal and breast cancer. Recognized for her 23-year commitment, she has received accolades such as the Kiwibank 2024 New Zealand Local Hero of the Year Te Pou Toko o te Tau medal and the Bridget Robinson Award for exceptional contributions to translational cancer research.

In her role at Otago University's He Taonga Tapu Cancer Society Tissue Bank, she manages all aspects, from collection to distribution, and has built a unique Aotearoa-specific collection from over 14,500 cancer patient donations. Morrin's influence has extended globally through her role as the chair of the

Science Policy Advisory Committee of the International Society for Biological and Environmental Repositories and her past executive membership in the Australasian Biospecimens Network Association.

Her dedication to ethical and culturally sensitive biobanking practices is evident both in her professional pursuits and as a member of the University of Otago's Human Ethics Committee. Morrin's international recognition, numerous publications, and contributions to global conferences highlight her unwavering commitment to advancing scientific research with integrity and excellence.



**Rita Lawlor, PhD**

Rita is an associate professor in the Department of Engineering for Innovative Medicine and a fellow of Information Privacy from IAPP (International Association of Privacy Professionals) at the University of Verona. Rita is originally a Computer Science graduate with a doctorate in translational biomedical sciences in Oncological Pathology. Rita Lawlor is co-founder and vice director of the ARC-Net Applied Cancer Research Centre where she coordinates research activities and runs the cancer biobank.

Rita is a member of the management committee of ICGC-ARGO, the international cancer genome consortium project to accelerate research in genomics oncology, and is co-PI for the Italian-associated project on orphan tumors. She is a member of the steering committee of BC-NET (Biobank Cohort Network of Low Middle Income Countries) network of IARC (International Association for Research on Cancer). She is a former director of ISBER, International Society for Biological and Environmental Repositories ([www.isber.org](http://www.isber.org)), and currently vice chair of ISBER Science Policy Community of Practice. She is past president of ESBB, the European, Middle Eastern, and African Society for Biopreservation and Biobanking ([www.esbb.org](http://www.esbb.org)). She is currently on the board of the Italian Foundation for Pancreas Diseases (FIMP).

Her current research interests are in molecular diagnostic markers and therapeutic targets and the role of cancer heterogeneity and molecular characterization of samples in the application of personalized medicine.



**Annette Schmid, PhD**

Annette is a Sr Director Global Science Policy at Takeda where she focuses on policies related to biospecimen, data, AI and innovative scientific approaches in clinical trials. She has 25+ years of experience as a scientist in academia and industry, co-founded the [Science Policy Think Tank](#), has lead a number of industry fora, including [PINTAD](#) and [GBITR](#) and has been part of the [QIBA](#) steering committee and [FNIH](#) working groups with a particular interest in seeing appropriate innovation and industry consensus in clinical trial endpoints for novel treatments. Prior to her career in science, she worked for the German State Department mostly in cultural and legal affairs.

*Moderator: Mark Barnes, Ropes & Gray and MRCT Center*

### **Panel 3: Diversity Action Plans, Global Considerations**



**Cherie Butts, PhD**

Cherie is Medical Director (Therapeutics Development Unit) at Biogen and has held multiple roles since joining the organization in 2012. She was clinical lead for Plegridy IM (peginterferon beta-1a, now approved for multiple sclerosis in the US and EU) and served on the inaugural cohort of Biogen's Portfolio Transformation team, where she led the probability of success theme and initiatives on change management, drug development for academic researchers, and project team learning. She is passionate about ensuring individuals from all backgrounds contribute to biomedical research and works with several organizations. This includes serving on Committees or as a Trustee/Board member of academic institutions (Beth Israel-Lahey Health/Harvard Medical School) and scientific organizations (American Association of Immunologists, Federation of American Societies for Experimental Biology, Keystone Symposia).



**Lola A. Fashoyin-Aje, MD, MPH**

Lola is a medical oncologist and Deputy Director in the Division of Oncology 3 (DO3) in the Office of Oncologic Diseases (OOD) at the Center for Drug Evaluation and Research- Food and Drug Administration (FDA). In this role, she provides clinical, scientific, and regulatory policy guidance and oversight to multidisciplinary teams reviewing drugs and biologics under development for the treatment of solid tumor malignancies. Dr. Fashoyin-Aje is also an Associate Director at the FDA Oncology Center of Excellence at the FDA, where she leads initiatives to address clinical and regulatory science and policy issues impacting oncology drug development. Prior to joining the FDA, Dr. Fashoyin-Aje completed her undergraduate and graduate training at Columbia University and Yale University, respectively, and received her M.D. degree from the University of Rochester School of Medicine and Dentistry. She completed postgraduate training in internal medicine and medical oncology at Johns Hopkins.



**LaShell Robinson, MS**

LaShell is the Head of Diversity, Equity & Inclusion in Clinical Research at Takeda. Her team works to ensure equitable trial access by fostering partnerships, implementing strategies, and embedding DEI into trial execution. LaShell has over a decade of experience in clinical research, supporting global clinical trial programs and serving as Clinical Operations Lead, Diversity & Inclusion in Clinical Trials. She is a proud alumna of Tuskegee University and has a Master of Science in Biomedical Engineering from the University of South Florida.

*Moderator: Barbara Bierer, MRCT Center*

## **R3 Topic 1: Update on the GDPR, including discussion of the EU/US data privacy Framework**



**Sara Berkson, JD**

Sarah is the Chief Privacy Officer and Global Head Privacy Counsel at Vertex Pharmaceuticals Incorporated. In this role, she leads the company's privacy program, overseeing and advising the company on data protection risks around the globe, including those in the clinical trial space. Sara also serves as the company's global Data Protection Officer. Sara has practiced in the privacy space since the advent of HIPAA in 2003, focusing on the implications of various international, federal, and state privacy laws on the healthcare and life sciences industries. She has spent time both in private practice (most recently prior to Vertex, at Verrill Dana, and before that, at Ropes & Gray) and in-house (at Genzyme Corporation), advising on a myriad of data protection issues that arise in the life sciences space, including those related to human subjects research, patient support programs, CRMs, and social media. Sara received her J.D. from Yale Law School and her A.B. in Psychology from Harvard University.



**Hannah Bracken, MS**

Hannah is a Policy Advisor focused on data flows between the European Union and the United States in the U.S. Department of Commerce's EU-U.S. Data Privacy Framework team. Hannah leads work on EU-U.S. health data flows and the EU data strategy for the Department of Commerce, as well as serving on TTC Working Group 6. Hannah previously worked on digital policy at the U.S. Mission to the European Union in Brussels, and before that, she was part of the digital economy team at Cullen International, where she focused on cybersecurity policy. Hannah received her master's degree from Sciences Po in Paris and her undergraduate degree from the University of Chicago. She also worked as a trainee lawyer in Paris for a year.





**Jennifer Couture, JD**

Jennifer is currently the Principal and Owner of JC Legal Services, LLC, where she provides General Counsel-as-a-service, as well as other consulting and contracting services to assist companies in their healthcare compliance and data protection/privacy. Previously, she was VP, Chief Privacy Officer of Alexion Pharmaceutical, Inc. | Alexion, AstraZeneca Rare Disease while simultaneously navigating progressive roles as Head Legal Counsel for PfG (Emerging Markets & Distributors), and Executive Director of Data Privacy. In this role, Jennifer created the Alexion Global Privacy Program, and subsequently led its integration following AstraZeneca's acquisition of Alexion while developing and rolling out AstraZeneca's new privacy model and corporate privacy programs. In addition to managing Global Privacy, Jennifer also led Alexion's Data Governance Committee, Records Management Program, as well as supported Alexion's Information Technology Department and Clinical Operations.

Prior, with Phillips, Jennifer was Director, Head of Privacy Risk Management and Compliance, and Senior Legal Counsel for Royal Philips. Here, she led a global network of Privacy Officers and Counsel in the company's first-ever centralized Privacy function. In addition, Jennifer led negotiations and managed regulatory inquiries and investigations. Earlier, she was Legal Counsel, North American Commercial & Privacy for Philip Electronics NA and Privacy Officer / Senior Manager for Philips Patient Care Clinical Informatics. Jennifer's earlier career included advancement across multiple roles with Fidelity Investments, including as Privacy Officer for Investment / Advisor Business, Risk, Legal & Compliance Director for the Consulting Actuary arm, as well as previous work as a transactional attorney in retirement business. Jennifer launched her career as a Legal Intern creating Elliot Hospital's HIPAA program, Intern with the Department of Health and Human Services, and Clinical Research / Project Assistant for OxiGene, Inc.

She holds a JD from Suffolk University Law School and a bachelor's degree in History and French from the University of Massachusetts at Amherst. She is a member of the Massachusetts Bar Association as well as Chief women's network, and National Charities League. She has been a Certified International Privacy Professional (CIPP) in the US and EU through IAPP since 2008.



**Victoria Perez Riu, JD**

Victoria has led the Privacy function at two top global pharmaceutical companies and is currently working as Chief Privacy Officer for Takeda. She is an international lawyer with multiple Privacy qualifications and has been working in privacy and data governance-related roles for many years. While she feels passionate about data protection, she has an eclectic background, having worked in both the public and private sectors, including the European Commission and in the Banking, Chemical and Pharmaceutical industries.

Victoria has a keen interest in shaping policies and regulations and interacting with regulators and policy makers. She is currently a Co-Chair at the EFPIA Data Governance Working Group, a board member of the International Pharmaceutical and Medical Device Privacy Consortium, and supporting two IMI projects associated with health research (H2O Health Outcomes Observatories and FACILITATE Framework for Clinical Trial Participants' Data Reutilization for a Fully Transparent and Ethical Ecosystem)

## **MODERATOR**



**David Peloquin, JD**

David practices law at Ropes & Gray LLP, where he is a member of the firm's healthcare group. He focuses his practice on advising academic medical centers, life sciences companies, and information technology companies on issues related to human subjects research and data privacy. He frequently writes and speaks on topics related to each of these areas and is a regular presenter at conferences and webinars of the American Health Law Association, the Association for the Accreditation of Human Research Protection Programs, the International Association of Privacy Professionals, and central and institution-specific institutional review boards. Outside of his law practice, David served until recently as a community member of the Institutional Review Board at Mass General Brigham in Boston. In recent years, David has spent considerable time advising clients on their response to the COVID-19 pandemic, including with respect to modifications to clinical research, implementation of telehealth technologies, and development and implementation of clinical diagnostic testing programs.

David has worked with MRCT Center since 2013. He has contributed to projects on data sharing, the return of research results to clinical trial subjects, and the impact of the European Union's General Data Protection Regulation (GDPR) on research. He has presented at the MRCT Center's Research, Regulatory, and Development Roundtable (R3) on topics including GDPR, secondary uses of health data for clinical trial recruitment purposes, legal and ethical issues that arise when a company or institution uses its own employees or students as research participants, and decentralized clinical trials.

David received his undergraduate degree from Carleton College, his law degree from the Yale Law School and clerked for Judge Diana E. Murphy of the United States Court of Appeals for the Eighth Circuit. Before attending law school, David worked as a project manager for Epic Systems, a manufacturer of electronic medical records.



## R3 Topic 2: Ensuring Integrity of Clinical Trial Data



**Nicholas Kenny, PhD**

Nick has over 25 years of experience in clinical development and consulting. He is passionate about rapidly moving compelling new science for unmet medical needs through the development process to arrive at early and innovative decisions.

Nick has been with the company since 2006. He grew and led the Oncology business until moving to the CSO role in 2018 where he now oversees the global **Medical Team**, the **Consortia Models** for Rare Diseases, Patient Voice, Biosimilars, **Global Safety/Pharmacovigilance**, and **Early Phase Clinics** and bioanalytics labs. Nick led and established our **Patient Diversity in Clinical Trials** initiatives and is an executive leader on the **DE&I Council**. Nick is Syneos Health's representative to the MRCT Steering Committee. His early career in biomedical research involved work academic centers in the UK, US and Canada. From 1991 – 1997 he held a faculty appointment at the University of Vermont Medical School. Cancer survivor (Hodgkin's Lymphoma). Past President, Board of Directors, Hospice of Wake County.



**Greg Licholai, MD**

Dr. Greg Licholai is Chief Medical & Innovation Officer at ICON plc where he has been President of Symphony Health and Care Innovations. Dr. Licholai is currently on faculty of Yale School of Management, Co-Director of the Center for Digital Health and has been visiting faculty at Harvard Business School. Previously, he was President of rare disease at Moderna Therapeutics, President and Chief Medical Officer at Castle Creek Pharmaceuticals, and partner at McKinsey & Co. where he led the healthcare data business. He was also a senior executive at Proteostasis, Amicus Therapeutics and Medtronic Neurological as well as venture investor for Domain Associates and was co-founder of Immunome Therapeutics.

Dr. Licholai attended Harvard Business School, Yale School of Medicine, Columbia University and Boston College. He trained in Neurological Surgery at the Brigham and Women's, Children's, and Massachusetts General Hospitals. He serves on multiple company and non-profit boards including advisor to the Clinical Trials Transformation Initiative (CTTI), a public-private partnership co-founded by Duke University and the Food and Drug Administration (FDA). Dr. Licholai writes about innovation in healthcare for Forbes, contributed to *Digital Therapeutics Strategic, Scientific, Developmental, and Regulatory Aspects*, and his textbook *Introduction to Medical Software: Foundations for Digital Health*,

Devices and Diagnostics is published by Cambridge University. He is on the board of the Epidermolysis Bullosa Research Partnership.



**Andy Lee, MS**

Andrew (Andy) Lee is Senior Vice President and Head of Global Clinical Trial Operations (GCTO). In this role, Andy leads and manages all operations related to the conduct of Merck's clinical trials, with particular focus on global in-patient clinical trials that are designed and executed to meet cost, speed and quality standards.

Andy is also responsible for the design and study and data management of clinical protocols in all regions and countries, as well as the tools, systems and processes used in clinical trial executions.

#### BACKGROUND

Andy joined Merck in September 2014 from Sanofi, where he served as Senior Vice President and Deputy Head of Clinical Sciences and Operations (CSO) and Head of the CSO Clinical Operations cluster. In addition to directing the CSO, Andy led the integration of Sanofi with Genzyme, where he had been Senior Vice President, Global Clinical Operations. Earlier in his career, he spent more than 16 years in a range of positions of increasing responsibility at Pfizer.

Andy holds leadership positions in several professional societies, including the role of Treasurer of TransCelerate Biopharma, Inc., a nonprofit organization that comprises the world's leading pharmaceutical and biotech companies. He received his M.S. in bioenergetics and physiology from Ball State University in Indiana, and two undergraduate degrees from Rhodes University in South Africa.

*Moderator: Mark Barnes, Ropes & Gray and MRCT Center*

## **Bioethics Collaborative- Gene Therapies: Probing the Ethics** **December 14, 2023**



**Erica Esrick, MD**

Erica is a pediatric hematologist at Dana-Farber/Boston Children's Cancer and Blood Disorders Center. Her primary clinical and clinical research interest is in hemoglobin disorders (sickle cell disease (SCD) and thalassemia). She is the clinical lead of the thalassemia program at Boston Children's Hospital (BCH). She is the clinical principal investigator of a sickle cell gene therapy trial at BCH and is also involved with multiple other hemoglobinopathy clinical research trials. Being engaged in the sickle cell gene therapy field in the early clinical years has afforded her the chance to connect with various stakeholders in the field, most importantly many patients and families, and also scientists, referring clinicians, patient/family advocacy groups, media, and industry colleagues.

After growing up in Evanston, Illinois, she attended Dartmouth College for undergraduate studies and then Harvard Medical School. She completed internship at Children's Memorial Hospital (now Lurie Children's) in Chicago and finished her pediatrics residency back in Boston at Boston Children's and Boston Medical Center. She completed a fellowship in pediatric hematology/oncology at Dana-Farber and Boston Children's. Erica lives with her husband, tween son, teen daughter, and poodle puppy in Jamaica Plain.



**Aric Parnes, MD**

Aric works as an Associate Medical Director in U.S. Hematology Medical Affairs at Vertex Pharmaceuticals focusing on CRISPR gene editing for sickle cell disease and thalassemia. A Michigander by birth, he attended the University of Michigan in Ann Arbor for college before going to Trinity College Dublin (Ireland) for medical school. He completed internship and residency in Internal Medicine at Dartmouth-Hitchcock Medical Center and fellowship in Hematology/Oncology at Yale New Haven Hospital and Dana-Farber Cancer Institute/Brigham and Women's Hospital in Boston. During fellowship, his research focused on genetic polymorphisms in myelodysplastic syndrome.

After completing his training, he joined a community practice in oncology in Milford, CT for two years before returning to Boston to join the faculty at Beth Israel Deaconess Medical Center as an oncology hospitalist and then Brigham and Women's Hospital as a staff hematologist and Assistant Professor of Medicine at Harvard Medical School. During his 10 years at BWH/DFCI/HMS, he served as Associate Director of the MGB/DFCI Hematology/Oncology Fellowship Program and Associate Director of the

Boston Hemophilia Center and has published in Blood, American Journal of Hematology, and New England Journal of Medicine.

During his time away from work, he can be found bringing his twin children to soccer games and skiing.



**Sonia Vallabh, PhD**

Sonia Vallabh co-runs a prion research laboratory at the Broad Institute of MIT and Harvard along with her husband, Eric Minikel. She earned her PhD in Biological and Biomedical Sciences from Harvard Medical School in 2019. Sonia and Eric left their previous careers to devote their lives to biomedical research after learning in 2011 that Sonia had inherited from her mother a mutation that causes genetic prion disease, a rapidly progressive and fatal dementia for which there is currently no treatment. Alongside drug discovery, Sonia is working toward new models to enable drugs to be tested for their ability to prevent or delay, as well as treat, neurodegenerative disease.



**Erin Ward, MEd, CAS**

Erin Ward is Co-Founder/President of MTM-CNM Family Connection, a non-profit for Myotubular & Centronuclear Myopathy. Erin has directed national conferences and led a Patient Listening Session and a Patient-Focused Drug Development Meeting with the FDA for this rare disease community. Recently, Erin co-led the creation of the international MTM-CNM Liver Collaborative Working Group. Holding a master's degree in education and a certificate of advanced study in counseling, Erin combines personal experiences as a mother to a son, who lived with Myotubular Myopathy, with her professional skills, to work towards improving care and patient-professional partnerships across both clinical and therapeutic drug development systems. Erin served as Associate Faculty for the Institute for Professionalism and Ethical Practice at Boston Children's Hospital and Harvard Medical School for over 15 years, facilitating medical education programs to enhance communication and shared decision-making among medical professionals and individuals living with complex medical conditions. She is a Patient Engagement Consultant for Boston Children's Hospital's complex spine and multidisciplinary trach team. Erin is an author, academic researcher, and presenter, most often on topics of patient engagement, complex care, and rare disease advocacy. Erin is a recipient of the Institute for Healthcare Improvement Sherman Award for Excellence in Patient Engagement. Erin continues her advocacy work in honor of her late son Will, who lived bravely every day with MTM, was an advocate for the community, and sadly passed away in November 2021 from complications of MTM.

## MODERATOR



### **Carolyn Chapman, PhD, MS**

Carolyn joined the MRCT Center in October 2023. She is a Member of the Faculty of the Department of Medicine at Harvard Medical School and Lead Investigator in the Division of Global Health Equity (DGHE), Department of Medicine, Brigham and Women's Hospital. Carolyn's work involves collaboration with diverse stakeholders to identify and address challenges in the research and development of precision medicine, including cell and gene therapies.

Prior to joining the MRCT Center, Carolyn was Faculty in the Center for Human Genetics and Genomics at NYU Grossman School of Medicine with a primary appointment as Research Assistant Professor in the Department of Population Health (Division of Medical Ethics). At NYU, she also served as Director of Research Ethics Education in the Translational Research Education and Careers (TREC) Unit of the Clinical & Translational Science Institute (CTSI) and as the organizer of and lecturer in the NYU component of the WCG International Fellows Program. In the past, she has also worked as an Associate/Lecturer and as Interim Associate Director for the Columbia Bioethics program; as a business strategy management consultant in the biopharmaceuticals industry at L.E.K. Consulting; at a start-up biopharmaceutical company, Aton Pharma; and as a freelance science/medical writer.

Carolyn graduated summa cum laude from Dartmouth College with a BA in Biology. She has a PhD in Genetics from Harvard University and an MS in Bioethics from Columbia University. She completed a postdoctoral fellowship in medical ethics at NYU Grossman School of Medicine and a Graduate Certificate in Survey Research at UConn's School of Public Policy.