Duke | MARGOLIS INSTITUTE for Health Policy

Building Quality into the Design and Conduct of Clinical Studies:

Integrating Quality by Design (QbD) and Risk-Based Monitoring (RBM) Approaches

Hybrid Public Meeting • National Press Club • Washington, DC

January 31, 2024 | 9:00 am – 4:20 pm ET

Meeting Background and Objectives:

Quality by design (QbD) and risk-based monitoring (RBM) approaches facilitate clinical trial modernization by enhancing efficiency without compromising data integrity or participant protections. The U.S. Food and Drug Administration (FDA) has encouraged the use of QbD and RBM through many outreach activities, such as co-hosting a public workshop in 2019 on implementing RBM approaches in clinical investigations. FDA has also recently published several guidance documents on these topics, such as E6(R3) Good Clinical Practice, A Risk-Based Approach to Monitoring of Clinical Investigations – Questions and Answers, and E8(R1) General Considerations for Clinical Studies. Despite the efforts of FDA and other interested parties, QbD and RBM approaches to plan and conduct clinical investigations have not been fully utilized. Additionally, as the COVID-19 pandemic necessitated changes to clinical trial design and conduct, recent experiences may help to inform the development of best practices for implementing QbD and RBM moving forward. This public workshop, convened by the Duke-Margolis Institute for Health Policy under a cooperative agreement with the FDA, is an opportunity to facilitate a discussion among the clinical trials community and interested parties about successes and challenges of integrating QbD and RBM into the design and conduct of clinical studies. The objectives of this meeting are to:

- Encourage the incorporation of QbD principles into the design and conduct of clinical studies, including the development of study protocols and workflow processes.
- Identify barriers to QbD and RBM implementation from the perspectives of patient advocates, sponsors, and clinical research organizations.
- Inform best practices for incorporating QbD and RBM approaches into the design and conduct of clinical studies.

Workshop Agenda

9:00 am **Welcome**

Mark McClellan, Duke-Margolis Institute for Health Policy

9:10 am **Opening Remarks**

Janet Woodcock, US Food and Drug Administration

9:20 am **Session 1: Risk-Based Approaches to Building Quality into the Design and Conduct of Clinical Investigations - Regulatory Perspectives of QbD and RBM**

Moderator: Jacqueline Corrigan-Curay, US Food and Drug Administration

Overview: This session will spotlight quality management principles in the design and conduct of clinical investigations with a focus on integrating QbD and RBM approaches based on recent FDA guidances. Panelists will discuss how regulators and clinical trial sponsors are approaching these principles.

Presentations, Moderated Panel Discussion, and Audience Q&A

Speakers/Panelists:

- **M. Khair ElZarrad**, US Food and Drug Administration
- **Fergus Sweeney**, Clinical Trial Expert, Retired
- **David Nickerson**, EMD Serono (Pharmaceutical Research and Manufacturers of America)
- **Kerstin Koenig**, GSK plc (European Federation of Pharmaceutical Industries and Associations)

10:35 am **Break**

10:45 am **Session 2: Optimizing Study Design and Setting the Stage for Efficient Study Conduct Through QbD: Successes and Challenges**

Moderator: Morgan Hanger, Clinical Trials Transformation Initiative

Overview: This session will highlight the successes and challenges of applying QbD in practice, with a focus on designing quality in study protocols, operations, and trainings. Panelists will discuss how to integrate a holistic approach to study design that includes patient perspectives, what critical-to-quality factors are necessary to identify and achieve study objectives, and how to evaluate and manage risk during the design phase.

Presentations, Moderated Panel Discussion, and Audience Q&A

Speakers/Panelists:

- **Kenneth Getz**, Tufts Center for the Study of Drug Development
- **Sameera Ibrahim**, Bristol Myers Squibb
- **Eda Baykal-Caglar**, Michael J. Fox Foundation
- **Leslie Sam**, Leslie Sam and Associates, LLC
- **Sabrina Comic-Savic**, Population Health Partners
- **Mokash Sharma**, Bristol Myers Squibb

12:15 pm **Lunch**

1:25 pm **Session 3: Translating QbD Principles to Risk-Proportionate Oversight Including RBM: Successes and Challenges**

Moderator: Laurie Muldowney, US Food and Drug Administration

Overview: This session will focus on identifying successes and challenges with implementing a risk-based approach to sponsor oversight of clinical trials, including trial monitoring and other trial-related activities performed by contracted service providers. Panelists will discuss the

adoption of RBM to optimize site performance through risk management while exploring artificial intelligence's (AI's) potential in advancing quality management.

Presentations, Moderated Panel Discussion, and Audience Q&A

Speakers/Panelists:

- **Nicole Stansbury**, Premier Research (Association of Clinical Research Organizations)
- **Patrick Nadolny**, Sanofi
- **Michael Walega**, Bristol Myers Squibb (PHUSE)
- **Kristin Stallcup**, Takeda
- **Steve Young**, CluePoints

2:55 pm **Break**

3:10 pm **Session 4: Next Steps for Implementing Quality Management of Clinical Investigations**

Moderator: Ann Meeker-O'Connell, US Food and Drug Administration

Overview: This panel discussion session will focus on determining what the immediate next steps are for sponsors, CROs and other interested parties to incorporate quality management into clinical studies. Panelists will identify best practices and needs that will facilitate increased adoption of quality management by interested parties presently and in the near future.

Moderated Panel Discussion and Audience Q&A

Panelists:

- **Danilo Branco**, Fortrea
- **Michael Torok**, Genentech
- **Kelsie Pearson**, Seattle Children's Hospital (Cystic Fibrosis Foundation)
- **Peter Stein**, US Food and Drug Administration

4:10 pm **Concluding Remarks**

Marianne Hamilton Lopez, Duke-Margolis Institute for Health Policy

4:20 pm **Adjournment**

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