DIA

Master Protocol Workshop

October 8-9 | Virtual

PROGRAM CHAIR

Daniel Millar, MBA
Senior Director, Strategic Business Transformation, Quantitative Sciences
Janssen Research & Development, LLC

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Director, Adaptive Trial Execution & Senior Statistical Scientist
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PROGRAM ADVISORS

Scott Berry, PhD
President and Senior Statistical Scientist
Berry Consultants LLC

Lisa LaVange, PhD
Professor and Associate Chair, Department of Biostatistics
University of North Carolina at Chapel Hill

Overview

The urgency to speed access to innovative therapies and vaccines has never been so evident as during the COVID-19 pandemic. With literally hundreds of potential medicines candidates under investigation, the ability to work efficiently and productively relies on open collaboration within frameworks that can answer multiple clinical questions in one overall trial structure. Master Protocols (MAPs) provide such frameworks and represent a paradigm shift in therapeutic research through an ongoing opportunity to more efficiently address a broader set of objectives than would be possible in a series of independent trials.

The concepts of Master Protocol trials have matured since the first oncology basket trials almost 20 years ago, and today we recognize multiple design types, such as adaptive platform and umbrella trials, for studying a wide array of indications. Examples include DIAN-TU (Alzheimer's Disease), REMAP-CAP (Community-Acquired Pneumonia), HEALY ALS (Amyotrophic lateral sclerosis), PrecISE (Asthma), and ACCORD (COVID-19), among others. As Master Protocols evolve and become more mainstream, they are presenting continual design, implementation, and operational learnings that can be built upon as the drug development paradigm shifts to one of collaboration and sharing.

Now in its third year, DIA's *Master Protocol Workshop* focuses on design, planning, implementation, start-up, and evolving operational best practices through the lens of Master Protocol trial stakeholders. Join industry, patient, regulatory agency, other government, and NGO representatives to examine the growing body of knowledge, experience, and resources available to better meet patient needs through successful Master Protocol trials.

Who Should Attend

Professionals within biopharmaceutical and medical device research, regulatory agencies, and patient organizations, who are involved in:

- Clinical Data Management, Clinical Operations, Clinical Research
- Good Clinical Practice
- Health Economics and Outcomes Research
- Medical Affairs
- Pharmacology
- Patient Engagement
- Regulatory Affairs
- Research and Development
- Rare/Orphan Diseases
- Statistics
- Strategic Planning
- Trial Design

Schedule At-A-Glance

DAY ONE | THURSDAY, OCTOBER 8

10:15-10:30AM	**Welcome and Opening Remarks**
10:30-11:30AM	**Session 1:** Master Protocols: Where Have We Been? How Far Have We Come?
11:30-11:45AM	Break
11:45AM-1:30PM	**Session 2:** Lessons Learned from Completed and Ongoing Master Protocols
1:30-2:30PM	Break
2:30-3:30PM	**Session 3:** Lessons Learned from Ongoing Master Protocol Platform Trial
3:30-3:45PM	Break
3:45-4:15PM	**Session 4:** Addressing Common Challenges—Clinicaltrials.gov Registration and IRB Reviews
4:15-4:25PM	Break
4:25-5:25PM	**Session 5:** Data and Information Sharing in Complex Innovative Trials

DAY TWO | FRIDAY, OCTOBER 9

8:30-10:00AM	**Session 6:** COVID-19 Learnings: Research Done Differently
10:00-10:15AM	Break
10:15-11:45AM	**Session 7:** Global Regulatory Landscape/Perspectives
11:45AM-12:15PM	Break
12:15-1:15PM	**Session 8:** Pushing the Boundaries of Master Protocol Studies: Frontiers of Design Innovation and Patient Engagement
1:15-2:00PM	**Closing Keynote Address**

Learning Objectives

At the conclusion of this activity, participants should be able to:

- Describe value drivers behind adaptive platform trials for key stakeholders (patient organizations, research sponsors, public institutions, regulatory agencies, and others) and for indications beyond oncology which would benefit from rapid development, the ability to study multiple therapies simultaneously, or the ability to study multiple patient subgroups simultaneously
- Discuss learnings that are emerging from completed and ongoing master protocol that can inform current master protocols experiencing challenges or facilitate the design and operationalization of new master protocol trials
- Summarize regional regulatory perspectives on adaptive platform trials and current progress toward global alignment

Continuing Education

DIA is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education. This session is designated for up to 10 contact hours or 1 continuing education units (CEU's). Type of Activity: Knowledge

ACPE credit is available if you attend the Master Protocol Workshop live October 8-9, 2020. Credit will not be awarded for watching the sessions On Demand post-conference.

ACPE CREDIT REQUESTS MUST BE SUBMITTED BY FRIDAY, NOVEMBER 20, 2020

Continuing Education Allocation

October 8 Day 1: Master Protocol Workshop: 5.25 contact hours or .525 CEUs Type of Activity: Knowledge, 0286-0000-20-128-L04-P

October 9 Day 2: Master Protocol Workshop: 4.75 contact hours or .475 CEUs Type of Activity: Knowledge, 0286-0000-20-129-L04-P

DIA is required by the Accreditation Council for Pharmacy Education (ACPE) to report pharmacy-requested CEUs through the CPE Monitor system. All ACPE-certified activity credit requests need to be submitted through DIA's My Transcript within 45-days post activity. If ACPE credit is not requested by Friday, November 20, 2020, the CEU request will not be transmitted through to the CPE Monitor. Pharmacists will need to provide their National Association of Boards of Pharmacy (NABP) e-Profile ID and date of birth (MMDD) to ensure the data is submitted to the ACPE and NABP properly. If you need to obtain your NABP e-Profile, please visit **www.cpemonitor.net**.

Continuing Education Credit and My Transcript

If you would like to receive a statement of credit for the days you attend the live virtual workshop, you must virtually attend the entire one or both days of the workshop, complete and return a CE Verification of Attendance Form (see instructions below), complete the post program evaluation and request CE credit online through My Transcript (see instructions below). Participants will be able to download a statement of credit upon successful submission of the credit request. My Transcript will be available for credit requests beginning **Friday, October 23, 2020**.

If you are claiming ACPE credit for this event you must:

1. Complete a Verification of Attendance Form
2. Send back to **NAEvents@DIAglobal.org** by **October 16, 2020**
3. Access your DIA account and select My Transcript to claim your ACPE credit, available on **Friday, October 23, 2020**

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DAY ONE | THURSDAY, OCTOBER 8

10:15-10:30AM

Welcome and Opening Remarks

Session Chair
Daniel Millar, MBA, Senior Director, Strategic Business Transformation, Quantitative Sciences Janssen Research & Development, LLC

Speaker
Robin Weinick, PhD, Senior Vice President and Managing Director, Americas and Global Program Officer DIA Global

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10:30-11:30AM

Session 1: Master Protocols: Where Have We Been? How Far Have We Come?

Abby Bronson, MBA, Vice President, Patient Advocacy and External Innovation, Edgewise Therapeutic

This session will build on the keynote and present the overall arc of master protocols, where we are in the trajectory of master protocols becoming mainstream and some of the key metrics that show their success. Starting with the early oncology trials and continuing with some of the newer rare disease trials, how and why master protocols are becoming more widely used will be discussed. The session will also cover master protocol adoption from the lens of patient advocacy as the drivers and beneficiaries of these innovative designs. A "glossary" of concepts and terms will be presented to foster a common language for master protocols and also to exemplify the importance of internal and external communication in planning and executing a master protocol.

History of Master Protocols and Where We Are
Scott Berry, PhD, President and Senior Statistical Scientist, Berry Consultants LLC

From the Lens of Patient Advocacy
Jane Perlmutter, Patient Advocate

A Glossary of Master Protocol Terms: What They Are and Why They Are Important
Cecile Spiertz, MSc, Senior Director, External Innovation Clinical Trial Platforms, Janssen Pharmaceutical, Netherlands

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11:30-11:45AM

Break

11:45AM-1:30PM

Session 2: Lessons Learned from Completed and Ongoing Master Protocols

Session Co-Chairs
Michelle Detry, PhD, Director, Adaptive Trial Execution & Senior Statistical Scientist, Berry Consultants LLC

AnnCatherine Downing, PharmD, Senior Research Advisor – Clinical, Eli Lilly and Company

This session will highlight lessons learned from multiple stakeholders across design, implementation, and data readout for a consortia-driven platform study. An ongoing, single-pharma sponsor basket trial will highlight lessons learned in building a business case, operational challenges, and planning for dissemination of results.

DIAN-TU Platform Trial: Lessons Learned Speakers
**this presentation will only be available live and not on demand*
Randall Bateman, MD, Charles F. and Joanne Knight Distinguished Professor of Neurology, Washington University School of Medicine

DIAN Platform Trial: Statistical Design
Scott Berry, PhD, President and Senior Statistical Scientist, Berry Consultants LLC

DIAN-TU Platform: Operational Complexities and Considerations
Susan Mills, Senior Director, Clinical Operations, Washington University School of Medicine, DIAN Trials Unit

IQVIA Perspective
Tina Gislimberti, MA, CNS Project Management, IQVIA

Perspective from Lilly
Phyllis Barkman Ferrell, MBA, Global Head, External Engagement, Alzheimer's Disease and Neurodegeneration, Eli Lilly and Company

Perspective from Roche
Geoffrey Kerchner, MD, PhD, Global Development Leader, F. Hoffmann-La Roche, Ltd., Switzerland

Speaker
Vivek Subbiah, MD, Executive Director, Medical Oncology Research, UT MD Anderson Cancer Center

Statistical Aspects of the ROAR Basket Trial
Kert Viele, PhD, Director and Senior Statistical Scientist, Berry Consultants

1:30-2:30PM **Break**

2:30-3:30PM **Session 3:** Lessons Learned from Ongoing Master Protocol Platform Trial

Session Co-Chairs
Michelle Detry, PhD, Director, Adaptive Trial Execution & Senior Statistical Scientist, Berry Consultants LLC

AnnCatherine Downing, PharmD, Senior Research Advisor – Clinical, Eli Lilly and Company

This session will present lessons learned from a newly initiated Master Protocol Trial regarding strategy and design choice, operational challenges and disclosure considerations, and the role of patients and advocacy groups in the platform trial development. This session will feature the experiences from the newly initiated HEALEY ALS (Amyotrophic Lateral Sclerosis) Platform Trial.

Speakers
Sabrina Paganoni, MD, PhD, Assistant Professor, Harvard Medical School, Healey Center for ALS at Mass General

Marianne Chase, Senior Director of Clinical Trial Operations, Neurological Clinical Research Institute, Massachusetts General Hospital

Panelists
Philip Green, ALS Patient Advocate, HEALEY ALS Platform Trial Patient Advisory Committee

Sundeep Sethi, MD, MBA, Vice President, Safety Operations, AbbVie, Inc.

Stephen Finger, PhD, ALS Patient, ALS Patient, Unaffiliated

3:30-3:45PM **Break**

3:45-4:15PM **Session 4:** Addressing Common Challenges—Clinicaltrials.gov Registration and IRB Reviews

Session Co-Chairs
Michelle Detry, PhD, Director, Adaptive Trial Execution & Senior Statistical Scientist, Berry Consultants LLC

AnnCatherine Downing, PharmD, Senior Research Advisor Clinical, Eli Lilly and Company

This session will have presentations on the unique considerations for registering a Master Protocol on clinicaltrials.gov and submitting for IRB reviews. The registering and review of these innovative designs requirements or educational materials for IRBs unfamiliar with Master Protocols.

Addressing Common Challenges: IRB Review
Heather Dobbins, PhD, ClinicalTrials.gov Lead Results Analyst, NCBI/NLM, National Institutes of Health (NIH)

Speaker
Lindsay McNair, MD, MPH, MSB, Chief Medical Officer, WIRB-Copernicus Group (WCG)

4:15-4:25PM **Break**

4:25-5:25PM **Session 5:** Data and Information Sharing in Complex Innovative Trials

Session Chair
Sudheer Doss, PhD, Chief Data Officer & Head of Business Development, Pancreatic Cancer Action Network

The Data and Information Sharing in Complex Innovative Trials session focuses on the balance across seamless trial execution, preservation of trial integrity, and maximization of research value from the data generated in complex innovative trials. Participants will hear about the challenges, risks, and solutions associated with two key aspects of data / information sharing:

1. Data and information availability / requirements for study execution

2. Sharing of data / information outside of study participants for secondary research

Data and Information Sharing in Complex Innovative Trials
Sudheer Doss, PhD, Chief Data Officer & Head of Business Development, Pancreatic Cancer Action Network

Master Protocols – What About the Data?
Shanna Allen, MPH, CCRA, Clinical Operations Regional Lead, Global Coalition for Adaptive Research

An Execution Platform for Implementing Innovative Trial and Project Designs
Raj Malathker, Senior Manager, Quantitative Sciences IT Janssen PharmaceuticalData and Information Sharing Considerations for Master Protocol Trials

Data and Information Sharing Considerations for Master Protocol Trials
Michelle Detry, PhD, Director, Adaptive Trial Execution & Senior Statistical Scientist, Berry Consultants LLC

DAY TWO | FRIDAY, OCTOBER 9

8:30-10:00AM **Session 6:** COVID-19 Learnings: Research Done Differently

Session Chair
Daniel Millar, MBA, Senior Director, Strategic Business Transformation, Quantitative Sciences, Janssen Research & Development, LLC

The value of Master Protocols has become better understood and accepted through unprecedented multi-stakeholder response to the COVID-19 pandemic. Over 300 clinical studies were set up in record time, including several Master Protocols for both therapeutics and vaccines. Suddenly, the ability to form productive consortia became a critical path challenge to implement quantitative innovations provided by Master Protocols. This session will focus on the underlying trends transforming Master Protocol studies and clinical research.

Speakers
Pamela Tenaerts, MD, MBA, Executive Director, Clinical Trials Transformation Initiative (CTTI)

Lennie Derde, MD, PhD, Intensivist and Clinician Researcher, UMC Utrecht, Netherlands

Catalyzing Innovation Through Collaboration
Janice Chang, Senior Vice President, Global Operations, TransCelerate Biopharma Inc.

Speaker
Lisa LaVange, PhD, Professor and Associate Chair, Department of Biostatistics, University of North Carolina at Chapel Hill

When Time Matters Most: Converting a Traditional Study into a Master Protocol in One Week
Tobias Mielke, Scientific Director, Janssen Pharmaceutical

10:00-10:15AM **Break**

10:15-11:45AM

Session 7: Global Regulatory Landscape/Perspectives

Session Chair

Daniel Millar, MBA, Senior Director, Strategic Business Transformation, Quantitative Sciences, Janssen Research & Development, LLC

The unprecedented COVID-19 experience accelerated the call for Master Protocols from global regulators, as a means of generating timely and substantial evidence to help patients and to utilize research sites efficiently. This session will focus on how regulatory views are evolving and will address important challenges to be addressed.

Speaker

Dionne Price, PhD, Director, Division of Biometrics IV, Office of Biostatistics, OTS, CDER, FDA

A European Regulator's View on Master Protocols

Benjamin Hofner, PhD, Head of Section Biostatistics, Paul-Ehrlich-Institut, Germany

Brazilian Experience with Master Protocols

Gustavo Mendes Lima Santos, General Manager of Medicines and Biological Products, ANVISA, Brazil

11:45AM-12:15PM

Break

12:15-1:15PM

Session 8: Pushing the Boundaries of Master Protocol Studies: Frontiers of Design Innovation and Patient Engagement

Session Co-Chairs

Amy Burd, PhD, Vice President, Research Strategy, Leukemia & Lymphoma Society

Kimberly Fisher, MA, Project Manager for Master Protocol Project, Clinical Trials Transformation Initiative (CTTI)

Optimizing the use of master protocol studies will require the clinical trials research community to (1) advance the exploration of new intervention modalities and (2) critically assess strategies to increase the inclusion of patient populations who currently sit at the margins of complex innovative trial design. In a series of lightening round presentations, experts will challenge assumptions about the types of problems and questions master protocol studies can be designed to address. Specific topics will include advancing the exploration of combination therapies and engaging underrepresented patients in the US and low and middle income countries.

Adaptive Bayesian Platform Trials

Donald Berry, PhD, Professor, Department of Biostatistics, M.D. Anderson Cancer Center

Master Protocols as a Measure to Improve Healthcare Outcomes in Low- and Middle-Income Countries

Jay Park, MSc, PhD, Director of Trials Research, Cytel, Canada

Engaging Racial and Ethnic Minorities in Master Protocol Studies

Christopher Flowers, MD, MS, FASCO, DrMed, Department Chair/Division Head Ad Interim, University of Texas MD Anderson Cancer Center

1:15-2:00PM

Closing Keynote Address

Session Chair

Daniel Millar, MBA, Senior Director, Strategic Business Transformation, Quantitative Sciences, Janssen Research & Development, LLC

"Master Protocols to Study Multiple Therapies, Multiple Diseases or Both", the seminal paper authored by Dr. Janet Woodcock and Dr. Lisa M. LaVange in 2017, highlighted the need to adopt these efficient, collaborative designs to meet the needs of patients more rapidly and cost-effectively. Today, the COVID-19 pandemic serves as an urgent prompt for building a clinical trial infrastructure that can rapidly repurpose itself to meet pressing needs. Join us for Dr. Woodcock's closing keynote, as she reflects on how the increasing adoption of master protocols will be critical to the reinvention of clinical research.

Speaker

Janet Woodcock, MD, Director, Center for Drug Evaluation and Research (CDER), FDA

2:00PM

Conference Adjourns