DIA

Master Protocol Workshop

October 29-30 | Hyatt Regency Bethesda | Bethesda, MD

PROGRAM CHAIR

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Senior Director, Strategic Business Transformation, Quantitative Sciences
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Victoria Manax Rutson, MD
Chief Medical Officer
Pancreatic Cancer Action Network

Rui (Sammi) Tang, PhD
Senior Director, Head of Biostatistics Department Leading the Biostats and Programming Team
Servier Pharmaceuticals

Overview

This year's DIA *Master Protocol Workshop* focuses on Adaptive Platform Trials (APTs), powerful trials that study multiple therapies with multiple patient populations at once. Join industry, patient, regulatory agency, and other government experts to explore funding and start-up considerations, regulatory engagement, and key operational concerns through the lens of four different APT implementation models. A panel of global regulators will share their perspectives on the implementation of these trials in their regions, application to a wide range of therapeutic areas beyond oncology, and progress toward global regulatory alignment.

Highlights

- Background on Master Protocols: a brief but important view of the history and important prior work, current state of the science, and when common types of master protocols can best be applied, with special focus on adaptive platform trial designs (APTs)
- Case studies of four models of APTs – patient organization, single company, multiple company, and public/government organized
- Examination of funding, start-up, regulatory, and operationalization considerations and best practices emerging from experience to date
- Global regulators' discussion on interest, challenges, and utilization of these trial designs within their regions: Europe, Asia, and US views

Who Should Attend

Professionals involved in:

- Biostatistics, Including Adaptive Design and Bayesian Statistics
- Clinical Research
- Research and Development
- Trial Design
- Clinical Operations
- Therapeutic Area Development, Management, and Operationist
- Rare disease, Oncology, Immunology, Alzheimer's disease
- Medical Affairs
- Medical Science Liaisons
- Medical Writing
- Regulatory Affairs

Schedule At-A-Glance

DAY ONE | TUESDAY, OCTOBER 29		ROOM
12:00-5:00PM	Registration	Regency Foyer IV
1:00-1:15PM	**Welcome and Opening Remarks**	Regency III
1:15-1:45PM	**Keynote Address:** PATIENTS FIRST: Integrated Research Platforms	Regency III
1:45-2:30PM	**Session 1:** Creating a Common Understanding of Master Protocols	Regency III
2:30-3:00PM	Refreshment and Networking Break	Regency IV
3:00-4:30PM	**Session 2:** Models for Implementing Platform Trials from the Lens of Patient Advocacy	Regency III
4:30-6:00PM	**Session 3:** Master Protocols With a Single Sponsor	Regency III
6:00-7:00PM	Networking Reception	Regency IV

DAY TWO | WEDNESDAY, OCTOBER 30		ROOM
7:00AM-4:00PM	Registration	Regency Foyer IV
7:15-8:15AM	Continental Breakfast and Networking	Regency IV
8:15-8:20AM	**Welcome to Day Two**	Regency III
8:20-10:00AM	**Session 4:** Global Regulatory Perspective	Regency III
10:00-10:30AM	Refreshment and Networking Break	Regency IV
10:30AM-12:00PM	**Session 5:** Multi-Company, Adaptive Platform Trials and Integrated Research Platforms: Clinical Research of the Future	Regency III
12:00-1:00PM	Luncheon and Networking	Regency IV
1:00-3:00PM	**Session 6:** Considerations for Publicly Funded Master Protocols with Research Objectives	Regency III
3:00 3:30PM	Open Discussion	Regency III
3:30-4:00PM	**Closing Remarks:** Where Do We Go From Here	Regency III

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) including how they may benefit and contribute to trial start up
- Discuss how stakeholder groups have successfully organized and started platform trials within common models of collaboration (patient organization driven, single company sponsor, multiple company sponsor, and government/public institution driven), addressing key elements such as funding, governance, and regulatory engagement paths
- Summarize regional regulatory perspectives on adaptive platform trials and current progress toward global alignment
- Discuss emerging best practices for key operational components that are critical success factors for platform trials, such as data sharing and network building

DIA is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education. This session is designated for up to 10.25 contact hours or 1.025 continuing education units (CEU's). Type of Activity: Knowledge

ACPE CREDIT REQUESTS MUST BE SUBMITTED BY DECEMBER 14, 2019

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 December 14, 2019, 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**.

DIA has been accredited as an Authorized Provider by the International Association for Continuing Education and Training (IACET).

As an IACET Authorized Provider, DIA offers CEUs for its programs that qualify under the ANSI/IACET Standard. DIA is authorized by IACET to offer 1 CEUs for this conference. Participants must complete the entire conference in order to be able to receive an IACET statement of credit. No partial credit will be awarded.

Continuing Education Credit Allocation

Day One: 4.25 contact hours or .425 CEUs, UAN: 0286-0000-19-071-L04-P

Day Two: 6 contact hours or .6 CEUs, UAN: 0286-0000-19-072-L04-P

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DAY ONE | TUESDAY, OCTOBER 29

12:00-5:00PM — **Registration**

1:00-1:15PM — **Welcome and Opening Remarks**

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

1:15-1:45PM — **Keynote Address:** PATIENTS FIRST: Integrated Research Platforms

Michael Krams, MD, Vice President, Global Head of Quantitative Sciences, Janssen R and D, Johnson and Johnson

1:45-2:30PM — **Session 1:** Creating a Common Understanding of Master Protocols

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

The list of publications on master protocols continues to expand, in parallel with expanding interest from diverse stakeholders to utilize master protocols to reach for the vision of patient-centric R&D and transform the model for drug development. With the expansion, diversity and sometimes confusion can emerge with respect to terminology, priority objectives, and implications. This session will focus on explaining the fundaments, present findings from a landscape analysis and present other original research which targets making design and implementation of master protocols easier.

Creating a Common Understanding of Master Protocols
Kimberly Fisher, MA, Project Manager for Master Protocol Project, Clinical Trials Transformation Initiative

Nicholas Richardson, DO, MPH, Medical Officer, Division of Hematology Products, CDER, FDA

2:30-3:00PM — **Refreshment and Networking Break**

3:00-4:30PM — **Session 2:** Models for Implementing Platform Trials from the Lens of Patient Advocacy

Session Co-Chairs
Abby Bronson, MBA, Senior Vice President of Research Strategy, Parent Project Muscular Dystrophy

Victoria Manax Rutson, MD, Chief Medical Officer, Pancreatic Cancer Action Network

Patients and patient advocacy groups are often at the heart of creating patient-centric improvements in developing therapeutics for their community. Increasing patient groups are driving innovation through the platform trial concept, especially in rare diseases, where the efficiencies of platforms are needed. But what should an advocacy organization focus on as it explores the feasibility of a platform trial? Through a case study and discussion, this session will explore key topics to operationalize a platform trial as seen through the lens of patient advocacy.

Victoria Manax Rutson, MD, Chief Medical Officer, Pancreatic Cancer Action Network

Value Drivers of Platform Trials - Who Might Fund?
Mark Trusheim, MSc, Researcher, Massachusetts Institute of Technology, Sloan School of Management,

Platform Trial Data Sharing Agreements
Sudheer Doss, PhD, Chief Data Officer, Pancreatic Cancer Action Network

Abby Bronson, MBA, Senior Vice President of Research Strategy, Parent Project Muscular Dystrophy

4:30-6:00PM **Session 3:** Master Protocols With a Single Sponsor

Session Co-Chairs
MaryAnn Morgan-Cox, PhD, Senior Director, Immunology Design Hub, Eli Lilly and Company

Rui (Sammi) Tang, PhD, Senior Director, Head of Biostatistics Department Leading the Biostats and Programming Team, Servier Pharmaceuticals

In contrast to traditional trial designs where a single drug is tested in a single disease population in one clinical trial, master protocols use a single infrastructure, trial design, and protocol to simultaneously evaluate multiple drugs and/or disease populations in multiple sub-studies, allowing for efficient and accelerated drug development. Although historically the master protocol concept has been utilized across multiple institutions or sponsors, master protocols are beginning to extend into single company sponsorship; this more discrete setting requires special attention with respect to operational, regulatory, and methodological considerations. This session invites industry experts who have identified and overcome trial complexities associated with simultaneous/concurrent evaluation within a single sponsor setting to share key learnings and best practices. We hope these case studies offer valuable information and support for companies that consider internal development of master protocols.

Basket Trial for Rare Dermatological Disorders
MaryAnn Morgan-Cox, PhD, Senior Director, Immunology Design Hub, Eli Lilly and Company

Innovative Approaches to Master Protocols in I/O
Rui (Sammi) Tang, PhD, Senior Director, Head of Biostatistics Department Leading the Biostats and Programming Team, Servier Pharmaceuticals

Master Protocols in Practice: Tumor Agnostic Approvals
Nora Ku, MD, Executive Medical Director, LOXO Oncology

6:00-7:00PM **Networking Reception**

DAY TWO | WEDNESDAY, OCTOBER 30

7:00AM-4:00PM **Registration**

7:15-8:15AM **Continental Breakfast and Networking**

8:15-8:20AM **Welcome to Day Two**

8:20-10:00AM **Session 4:** Global Regulatory Perspective

Session Co-Chairs
Daniel Millar, MBA, Senior Director, Strategic Business Transformation, Quantitative Sciences, Janssen Research and Development, LLC

Zoran Antonijevic, MS, Head of Biometrics, MedSource

MaryAnn Morgan-Cox, PhD, Senior Director, Immunology Design Hub, Eli Lilly and Company

Regulatory acceptance is critical to make novel development approaches such as platform trials viable for drug developers and patients. Furthermore, scaling novel approaches to become mainstream requires moving from acceptance to active championship and engagement across stakeholder groups, as well as, a global perspective. In this section, we will explore the global regulatory environment by surveying the interest, openness, and critical challenges foreseen by influential, global regulators with respect to master protocols/platform trials as a patient-centric innovation. Regulatory perspective will span biometrics, clinical review and clinical trial operations.

European CTFG Perspective On: Complex Clinical Trials
Ditte Zerlang Christensen, PhD, Senior Regulatory Assessor, Danish Medicines Authority, Denmark

Peter Stein, MD, Director, Office of New Drugs, CDER, FDA

Catherine Njue, PhD, Biostatistics Advisor-Clinical Trials, Office of Biostatistics, Centre for Regulatory Excellence, Statistics and Trials (CREST), Biologics and Genetic Therapies Directorate, Health Products and Food Branch, Health Canada

10:00-10:30AM — **Refreshment and Networking Break**

10:30AM-12:00PM — **Session 5:** Multi-Company, Adaptive Platform Trials and Integrated Research Platforms: Clinical Research of the Future

Session Co-Chairs
Daniel Millar, MBA, Senior Director, Strategic Business Transformation, Quantitative Sciences, Janssen Research and Development, LLC

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

Master protocols offer substantial opportunities to achieve efficiencies in clinical development both operationally and inferentially. Maximizing these benefits and delivering on the promise of patient-centric R&D, requires going beyond the capability of any one company or organization to ensure patients have the best opportunity to participate in a trial that may benefit them and promising therapies or futile endeavors can be identified quickly, allowing reallocation of scare resources to a more fruitful direction. This section will provide updates on a multi-company, public-private partnership, adaptive platform trial, IMI EPAD (European Prevention of Alzheimer's Dementia Consortium), including learnings related to trial design, stakeholder collaboration and the business model for sustainability.

Kristy Draper, **PhD**, Global Trial Lead for European Prevention of Alzheimer's Dementia Consortium, The University of Edinburgh, United Kingdom

Overview of IMI EU-PEARL
Esther Arevalo, **MS, Msc**, EU-PEARL Project Management Strategy Unit, Vall d'Hebron Institute of Research (VHIR), Spain

12:00-1:00PM — **Luncheon and Networking**

1:00-3:00PM — **Session 6:** Considerations for Publicly Funded Master Protocols with Research Objectives

Session Co-Chairs
Lisa Lavange, PhD, Professor and Associate Chair, Department of Biostatistics, UNC

Rui (Sammi) Tang, PhD, Senior Director, Head of Biostatistics Department Leading the Biostats and Programming Team, Servier Pharmaceuticals

In this session, we will explore the use of master protocols in research studies funded by government agencies such as the National Institutes of Health or from other sources of public funding. There are several differences between these studies and their industry-funded counterparts including negotiations with stakeholder groups during organization and implementation, trial governance, data use and sharing, and safety reporting, have an impact on the desirability and feasibility of conducting a master protocol and must be taken into consideration in study planning. Some of these differences will be examined through a case study on the NHLBI PrecISE (Precision Interventions for Severe Asthma) study, which includes a novel precision medicine component. Motivations to pursue a master protocol in severe asthma, the study design, and clinical perspectives will be discussed.

Publicly Funded Master Protocols Compared to Their Industry Counterparts
Lisa LaVange, PhD, Professor and Associate Chair, Department of Biostatistics, UNC

The Rationale for NHLBI Funded Master Protocols
Patricia Noel, **PhD**, Project Officer for PrecISE, Lung Division, NHLBI, NIH

Statistical Innovation in Trial Design for Master Protocols
Anastasia Ivanova, PhD, Professor, Department of Biostatistics, University of North Carolina at Chapel Hill, Coordinating Center PI for PrecISE

3:00-3:30PM — **Open Discussion**

3:30-4:00PM — **Closing Remarks:** Where Do We Go From Here

Session Chair
Abby Bronson, MBA, Senior Vice President of Research Strategy, Parent Project Muscular Dystrophy