So You Want to Start a Master Protocol Trial...

December 10, 2018
2-3 pm ET
Tanisha Carino

**Executive Director**

*FasterCures, a Center of the Milken Institute*
10,000 DISEASES.
ONLY 500 TREATMENTS.
WE HAVE WORK TO DO.
Webinars

Purpose

• **Real-time** sharing of ideas, best practices, trends, and lessons learned
• **Amplifies** meaningful solutions, productive tools, and encourages action needed to spur medical progress

Topics

• Patient-Centered Measurement
• Patient-Focused Drug Development
• Innovations in Clinical Trials
• Venture Philanthropy

Who’s logged on?

~300 registrants from:

- **Nonprofit** 50%
- **Academia** 4%
- **Pharmaceutical** 14%
- **Biotechnology** 6%
- **Government** 5%
- **Consulting** 3%
- **Other** 18%
Anna Barker

Co-Director, Complex Adaptive Systems; Director, National Biomarker Development Alliance; Professor, School of Life Sciences Arizona State University

MODERATOR
“So You want to Start a Master Protocol Trial”

FasterCures Webinar
December 10, 2018

Anna D. Barker, Ph.D.
Co-Director, Complex Adaptive Systems Initiative
Director, National Biomarker Development Alliance
Professor, School of Life Sciences
Arizona State University
Senior Fellow, Faster Cures, Center of the Milken Institute
Clinical Trials: Massive Attrition, Long Duration, High Costs – How Can We Transform this System?

5-10,000:1 chance of success  12-15 Years  ~ US$ 2.0 B

“Precision Medicine Requires a New Model of Drug Development”
Master Protocols – What are they and why are they important?

Master Protocol: Amended from FDA (Draft oncology guidance, 2018)

“……a master protocol is defined as a protocol designed with multiple sub-studies (arms), which may have different objectives and involves coordinated efforts to evaluate one or more investigational drugs (or biologics, combinations) in one or more (diseases) disease subtypes within the overall trial structure” *

Which Means

“efficiently answering multiple questions faster and more cost effectively under a single trial structure”

Types of Master Protocols: Basket, Umbrella and Platform Trials
(Lisa LaVange will explain)

*FDA Guidance's/UC M621817
Adaptive Platform Trials

**Patient Population**

- Stratify Based on Biomarkers

**Adaptive Randomization**

- Control Arm
- Experimental Arm A
- Experimental Arm B
- Experimental Arm C
- Experimental Arm D

Interim Analysis

Determine Efficacy of Treatments in Different Biomarker Signatures

Continue Trial, Adjust Adaptive Randomization So Patients Are More Likely to Be Assigned to Effective Arms
ISPY is an adaptive platform master protocol trial. Agents (including combinations) are tested in subsets of patients based on biomarker signatures, with pathologic complete response (PCR) used as the endpoint. Successful agents “graduate” and are returned to the company with a probability estimate of their potential success in a small phase 3 trial.

- ISPY-2 (in its eight year) has treated over 1300 patients and tested 17 agent
- Seven drugs have “Graduated” 7, 1 “graduate” received accelerated approval and 1 received “breakthrough designation
- Trial has shown that pathologic complete response is an excellent surrogate endpoint for long term survival – regardless of treatment
- PCR was approved by FDA as a surrogate endpoint in 2015
Current Framework (I-SPY2)

**Screening**
- MRI
- Biopsy
- Blood Draw

**Adaptive Randomization**

**Subtype**

**T0**
- HR+, HER2, MP++

**T1**
- Paclitaxel* (control arm)
  - 12 weekly cycles

**T2**
- Invest. Agent† 1 ± Paclitaxel*
  - 12 weekly cycles

**T3**
- Invest. Agent† 5 ± Paclitaxel*
  - 12 weekly cycles

**T5**
- Surgery

**Biomarker-rich protocol**

* Patients who are HER2+ may also receive trastuzumab (Herceptin)
† An investigational combination of one or more agents may be used to replace all or some of the standard therapy

Attribution, Laura Essermam
GBM AGILE Design (Adaptive Platform Master Protocol)

- New patient accrues; assess subtype
- Update patient outcome data
- Update longitudinal model
- Randomize to Exp. or control arm
- Determine randomization probabilities within each subtype
- Update probabilities Stage 1 arm > control for each subtype
- Add new experimental arms accrual permitting
- Calculate prob Stage 1 arm > ctl in each signature
- Decision rules for Stage 1 arms
- Stop futility
- Stop max n
- Graduate
- Continue in Stage 1
- Enter Stage 2
- Stop accrual

GBM AGILE Trial
Adaptive Platform Master Protocol
Seamless 2-3 Transition (via Algorithm)
Advantages of Master Protocol Trials – Focus on Adaptive Platform Trials

- Truly patient-focused at every level (many arms, adaptive randomization, serial review of data and common control) – and supports best and most efficient use of patient resources
- Enables simultaneously testing many new therapies in disease subtypes-incorporates biomarkers for hypothesis testing
- Utilizes adaptive randomization designs to more quickly identify effective agents and drop ineffective agents (e.g., ISPY 2 and GBM AGILE designs)
- Utilizes the best indicators of treatment effects for the disease
- When performed on a global scale incorporates genetic diversity
- May offer innovative path to registration trials – reduced time and costs
- For platform trials, creates a standing “infrastructure (harmonization of imaging, biospecimens, data management) – reduces costs and cycle time

Serve as continuous learning systems–learns and evolves from every patient
Lisa LaVange

Professor and Associate Chair, and Director
Collaborative Studies Coordinating Center,
Department of Biostatistics, Gillings School
of Global Public Health, University of North
Carolina
MASTER PROTOCOLS IN COLLABORATIVE RESEARCH

Lisa LaVange, PhD
Professor and Associate Chair

FasterCures Webinar
December 10, 2018

GILLINGS SCHOOL OF GLOBAL PUBLIC HEALTH
Biostatistics
Motivation: Precision Medicine

• Personalized medicine is getting the right product to the right patient (and in case of a drug or biologic) at the right dose

• Key challenge is to identify targeted subgroups at the right stage

• As medicines (or their targets) become more precise, traditional clinical trials become more difficult to conduct and/or less efficient

• Patients may need to be screened (sequentially) for several trials before finding an eligibility match

• Collaborations such as master protocols are of increasing interest, as a result
Motivation: Other Settings

• Collaborative efforts such as master protocols have advantages in non-precision medicine settings as well

• Example: rare diseases
  • Patients are scarce; multiple sponsors often compete for patients in concurrently active development programs

• Example: large safety trials
  • Usually involve ruling out excessive risk of rare events, requiring large sample sizes of high-risk patients (e.g., cardiovascular safety trials of diabetes drugs)
  • Sharing control groups within a master protocol could save time and resources, and reduce the number of at-risk patients on placebo

• Within a single company, multiple candidate drugs in the same therapeutic area may be studied under a master protocol for coordinated, concurrent development
Master Protocols

- Multiple diseases, multiple patient subgroups (biomarker-defined), and/or multiple therapies studied under one, over-arching protocol

- Also known as:
  - Umbrella or platform trials: one disease, multiple drugs (example: NCI Match)
  - Basket trials: one drug, multiple disease cohorts (example: B225 trial of imatinib)

- Exploratory: Identify best treatment for biomarker-defined patient subgroup (example: I-SPY II)

- Confirmatory: Evaluate different therapies relative to control for a single disease in parallel (example: Lung MAP)
Master Protocols

- Capitalize on similarities among trials and shared infrastructure to realize efficiencies
- Need regulatory buy-in
- Need sponsors willing to test drugs in collaboration with others
- FDA’s advocacy has had an influence
  - Support of programs like I-SPY 2, Lung MAP, PREVAIL II, ADAPT, DIAN-TU
  - Goal is to provide data to support regulatory approval of new drug or indication
  - FDA approval of study design usually applies to all sponsors contributing drugs
- Patient advocacy groups are often the catalyst for the needed collaboration
High-quality evidence is what we use to guide medical practice. The standard approach to generating this evidence—a series of clinical trials, each investigating one or two interventions in a single disease—has become ever more expensive and challenging to execute. As a result, important clinical questions go unanswered. The conduct of “precision medicine” trials to evaluate targeted therapies creates challenges in recruiting patients with rare genetic subtypes of a disease. There is also increasing interest in performing mechanism-based trials in which eligibility is based on criteria other than traditional disease definitions. The common denominator is a need to answer more questions more efficiently and in less time.

A methodologic innovation responsive to this need involves coordinated efforts to evaluate more than one or two treatments in more than one patient type or disease within the same overall trial structure. Such efforts are referred to as master protocols, defined as one overarching protocol designed to answer multiple questions. Master protocols may involve one or more interventions in multiple diseases or a single disease, as defined by current disease classification, with multiple interventions.
Master Protocols

Two avenues for innovation:

1. Establish a trial network with infrastructure in place to streamline trial logistics, improve data quality, and facilitate data sharing and new data collection

2. Develop a common protocol for the network that incorporates innovative statistical approaches to study design and data analysis
Infrastructure Advantages

- Centralized biomarker screening using common platform
  - More efficient for patients – send to sub-trial best suited for their biomarker profile

- Trial systems in place
  - Central randomization (e.g., via web portal)
  - Central electronic data capture system
  - In-network clinic personnel trained and experienced on existing systems

- Centralized governance structure
  - Use of central Steering Cmte, IRBs, standing DMC, adjudication cmtes, etc.

- Central labs, reading centers, etc., with QA oversight

- Common elements of trial protocols and common CRFs
  → Gain efficiencies in study-start-up and conduct, trial monitoring, and data close-out
Innovative Design Possibilities

- Adaptive randomization (response adaptive or covariate adaptive)
- Use of external or historical control data
  - In single-arm studies, or
  - In conjunction with concurrent controls (with 2:1 or higher) to increase power; potential adaptation
- Sharing of control groups – when it makes sense, e.g., within a specific pathway or biomarker-defined subgroup
- Model-based analysis methods (e.g., hierarchical Bayes) for pooled analysis of multiple diseases or tumor types, markers, body sites, etc.
- Precision medicine analysis to refine target subgroups
  - Adaptive enrichment
Adaptive Designs for Clinical Trials of Drugs and Biologics
Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the Federal Register of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the Federal Register.

For questions regarding this draft document, contact (CDER) Scott N. Goldie at 301-796-2055, or (CBER) Office of Communication, Outreach and Development, 800-855-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

September 2018
Clinical/Medical

Master Protocols: Efficient Clinical Trial Design Strategies to Expedite Development of Oncology Drugs and Biologics Guidance for Industry

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U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Oncology Center of Excellence (OCE)

September 2018
Procedural
Master Protocols – Lessons Learned

• Dynamic nature of master protocols and adaptive platform trials well-suited for the fast pace of precision medicine drug development

• Infrastructure development requires more resources up front, but with the potential to produce savings as trials continue

• Considerable investment required, also up front, to establish the collaboration among interested parties
  • Obtaining buy-in from pharmaceutical sponsors can be particularly challenging, due to competing commercial interests

• Involvement of patient advocacy groups (e.g., Friends of Cancer Research for Lung MAP) greatly enhances the ability to launch a master protocol in a timely fashion
Louis DeGennaro
President & CEO
The Leukemia & Lymphoma Society
Beat AML Umbrella Study for Newly Diagnosed AML

Louis J. DeGennaro, PhD
President & CEO
The Leukemia & Lymphoma Society
WHY A PRECISION MEDICINE AML TRIAL?

Most commonly diagnosed and most lethal adult leukemia

AML is a highly heterogeneous disease and we have substantial understanding of the underlying genetic mutations

Our hypothesis is that we can improve outcomes by matching patients to the increasing number of available targeted therapies
WHAT IS BEAT AML?

LLS convened partners with a goal of identifying more efficacious and better tolerated therapies

- Academic institutions
- Pharmaceutical Companies
- Regulatory agencies (FDA)
- Genomic vendors
- CRO and trial vendors

Trial seeks to identify impactful therapies or novel-novel combinations
KEY ELEMENTS OF BEAT AML TRIAL

Trial focuses on the patient

Looking for therapies with substantial clinical benefit

Rapidly adaptable to allow for changes that occur in the AML field (new knowledge, new agents, resistance mechanisms)

Committed to career development of junior investigators driving the individual studies
PRIMARY OBJECTIVES – MASTER TRIAL

1. To determine the feasibility of completing molecular, immunophenotypic, and/or biochemical studies in ≤ 7 calendar days

2. To determine the feasibility of assigning patients to sub-studies in this Master Protocol, based on these results

3. To determine the clinical efficacy of novel treatment strategies in each of the sub-studies
BEAT AML STUDY DESIGN

Patient Registration Consent → Bone Marrow Sample → Genomic Screening ≤ 1 Week (7 days) → Assign Treatment by Marker → Marker Positive → Targeted Agent or Combination → Initiation of Trial

Marker Negative → Novel Agent → Initiation of Trial

Primary Endpoint: CR; CRi
Collaboration is key
FEASIBILITY

Can we assign patients to therapy within 7 calendar days of samples arriving at reference lab?

<table>
<thead>
<tr>
<th>Assigned in 7 days</th>
<th>Not Assigned in 7 days</th>
<th>Total</th>
<th>% Assigned in 7 days</th>
</tr>
</thead>
<tbody>
<tr>
<td>273</td>
<td>12</td>
<td>285</td>
<td>95.8%</td>
</tr>
</tbody>
</table>

Answer: Yes!
# BEAT AML MASTER TRIAL CURRENT ENROLLMENT

**N=356**

66 Screen Fail
5 Awaiting Assignment

**N=285 Assigned Treatment**

<table>
<thead>
<tr>
<th>N (%)</th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>CBF</td>
<td>4 (1.4)</td>
<td></td>
</tr>
<tr>
<td>NPM1 w FLT3 ITD-</td>
<td>37 (13)</td>
<td></td>
</tr>
<tr>
<td>MLL</td>
<td>6 (2.1)</td>
<td></td>
</tr>
<tr>
<td>IDH2</td>
<td>32 (11.2)</td>
<td></td>
</tr>
<tr>
<td>IDH1</td>
<td>15 (5.3)</td>
<td></td>
</tr>
<tr>
<td>TP53</td>
<td>55 (19.3)</td>
<td></td>
</tr>
<tr>
<td>TP53 WT/Complex Karyotype</td>
<td>23 (8.0)</td>
<td></td>
</tr>
<tr>
<td>TET2/WT1</td>
<td>34 (11.9)</td>
<td></td>
</tr>
<tr>
<td>FLT3</td>
<td>19 (6.7)</td>
<td></td>
</tr>
<tr>
<td>Marker Negative</td>
<td>60 (21.1)</td>
<td></td>
</tr>
</tbody>
</table>

**N=146 Treated**

<table>
<thead>
<tr>
<th>N</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>CBF</td>
<td>2</td>
</tr>
<tr>
<td>NPM1 w FLT3 ITD-</td>
<td>10</td>
</tr>
<tr>
<td>MLL</td>
<td>1</td>
</tr>
<tr>
<td>IDH2</td>
<td>27</td>
</tr>
<tr>
<td>IDH1</td>
<td>1</td>
</tr>
<tr>
<td>TP53</td>
<td>19</td>
</tr>
<tr>
<td>TP53 WT/Complex Karyotype</td>
<td>2</td>
</tr>
<tr>
<td>TET2/WT1</td>
<td>23</td>
</tr>
<tr>
<td>FLT3</td>
<td>0</td>
</tr>
<tr>
<td>Marker Negative</td>
<td>61</td>
</tr>
</tbody>
</table>
PATIENTS NOT TREATED

- 139 patients (48.8%) not treated, most choosing other therapies
- At inception, the study began with only 3 sub-studies>
  increased options over time
- Treatment decision always guided by “what is best for the patient”

<table>
<thead>
<tr>
<th></th>
<th>Number of Patients</th>
<th>% Overall Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Died during 7 day</td>
<td>7</td>
<td>2.5</td>
</tr>
<tr>
<td>Alternative Treatment Prior to Assignment</td>
<td>20</td>
<td>7</td>
</tr>
<tr>
<td>Standard of Care</td>
<td>57</td>
<td>20</td>
</tr>
<tr>
<td>Alternative Trial After Assignment</td>
<td>26</td>
<td>9.1</td>
</tr>
<tr>
<td>Palliative Care</td>
<td>23</td>
<td>8.1</td>
</tr>
<tr>
<td>Not Specified</td>
<td>6</td>
<td>2.1</td>
</tr>
</tbody>
</table>
# BEAT AML ACTIVE STUDIES

<table>
<thead>
<tr>
<th>AML Subtype</th>
<th>Drug</th>
</tr>
</thead>
<tbody>
<tr>
<td>CBF</td>
<td>Samalizumab (CD200 Ab) + induction</td>
</tr>
<tr>
<td>NPM1 + FLT3-ITD</td>
<td>Entospletinib (Syk inhibitor) + induction (fit)</td>
</tr>
<tr>
<td></td>
<td>Entospletinib (Syk inhibitor) monotherapy (unfit)</td>
</tr>
<tr>
<td>MLL rearranged</td>
<td>Entospletinib (Syk inhibitor)</td>
</tr>
<tr>
<td></td>
<td>Entospletinib + Aza</td>
</tr>
<tr>
<td>IDH2 +</td>
<td>Enasidenib</td>
</tr>
<tr>
<td></td>
<td>Enasidenib + Aza</td>
</tr>
<tr>
<td>IDH1 +</td>
<td>Ivosidenib + Aza</td>
</tr>
<tr>
<td>TP53+</td>
<td>Entospletinib (Syk inhibitor) + Decitabine</td>
</tr>
<tr>
<td>TP53 - Complex Karotype (&gt; 3 abn)</td>
<td>Entospletinib (Syk inhibitor) + Decitabine</td>
</tr>
<tr>
<td>TP53+</td>
<td>Pevonedistat (Nedd8 inhibitor) + Aza</td>
</tr>
<tr>
<td>FLT3-ITD+ or FLT3-TKD +</td>
<td>Gilteritinib monotherapy or + Decitabine</td>
</tr>
<tr>
<td>Tet2/WTI</td>
<td>BI 836858 (CD33 Ab) + Aza</td>
</tr>
<tr>
<td>Marker Negative</td>
<td>BI 836858 (CD33 Ab) + Aza</td>
</tr>
</tbody>
</table>
INDIVIDUAL STUDY UPDATES

Phase 1b dose escalation with BI836858 + Azacitidine
(Abstract 4053, W Blum et al)
- Phase 1b completed
- Phase 2 recommended dose 80 mg

Phase 2 of Enasidenib in IDH2-mutant patients
(Abstract 287, E Stein et al)
- 44.4% ORR (12/27 patients)
- Phase 2 study expanded

Studies Discontinued:
- Phase 1 study of Samalizumab with standard 7 + 3 in CBF AML
CONCLUSIONS

• Implementation of a rapid treatment assignment umbrella study in elderly AML is feasible with 95% of patients assigned to treatment in ≤ 7 days

• Early death and disease progression prior to treatment assignment is uncommon

• Majority of patients assigned to protocol therapy proceed to trial (with increasing frequency as new protocols open)

• Promising efficacy observed in several of the treatment arms to date
ACKNOWLEDGEMENTS

• Patients and Families
• Dr. Amy Burd, Vice President, Research Strategy at LLS
• Syneos
• Foundation Medicine
• And the generous donors of the Leukemia & Lymphoma Society
Victoria Manax

Chief Medical Officer
Pancreatic Cancer Action Network
Precision Promise™ aims to **transform** clinical trials for pancreatic cancer

*PanCAN sponsored adaptive trial platform* with multiple study arms and control arms

*Clinical, molecular and outcomes data tracked longitudinally* and analyzed together across 14 sites

*Smaller sample size, shorter time to results*, re-randomization of patients, and flexibility to drop and add arms

Design pre-approved through discussions with FDA, including *two-stage design with graduation to registration-ready trial*
Why now and Why PanCAN?

Why now?

• The current clinical trial process in Oncology is *primed for disruption* and a *paradigm shift is required* to accelerate drug development

• Patients with Pancreatic Cancer have been *searching for innovative therapies* with minimal success *and require significant scientific advancements* to improve outlook

Why PanCAN?

• *PanCAN is uniquely positioned* to lead a collaborative model with a network of leading researchers, clinicians, trial experts, and drug developers

• PanCAN is a *trusted and unbiased stakeholder* utilizing a patient-centric approach to launch the first adaptive clinical trial platform for pancreatic cancer

• *The FDA recognizes the promise of adaptive clinical trial platforms* and believes patient advocacy organizations can help facilitate them
Precision Promise employs a unique business model and stakeholder partnerships

**Precision Promise: overall operating model**

<table>
<thead>
<tr>
<th>Operations</th>
<th>Infrastructure</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>IND holder:</strong> PanCAN</td>
<td>• Organizational structure for Precision Promise has a steering committee and six sub-committees to ensure efficiency</td>
</tr>
<tr>
<td><strong>CRO:</strong> Covance</td>
<td>• Each sub-committee has a chair, and the chairs make up the steering committee</td>
</tr>
<tr>
<td><strong>Statistical design:</strong></td>
<td></td>
</tr>
<tr>
<td><strong>Genomics sequencing &amp; analysis:</strong> TEMPUS</td>
<td></td>
</tr>
</tbody>
</table>

**Steering Committee**
- Diane Simeone, MD (Chair)
- Andrew Hendifar, MD
- Manuel Hidalgo, MD, PhD
- Anirban Maitra, MBBS
- Victoria Manax, MD
- Lynn Matrisian, PhD, MBA
- Vincent Picozzi, MD
- Sudheer Doss, PhD

**Sub-committees**
- Design
- Biomarker
- Supportive Care
- Arms Selection
- Operations
- Clinical Trial Consortium

**Pharma**
- Precision Promise Initiative requires pharma companies to partner

**Clinical trial consortium of medical centers selected through a competitive peer-review process:**

- Cedars-Sinai
- Dana-Farber Cancer Institute
- Fred Hutch Cures Start Here
- Memorial Sloan Kettering Cancer Center
- NYU Langone Medical Center
- UConnHealth
- UC San Diego Moores Cancer Center
- UCSF Helen Diller Family Comprehensive Cancer Center
- Perelman School of Medicine University of Pennsylvania
- The University of Chicago Medicine
- Washington University in St. Louis School of Medicine
- Johns Hopkins Medicine

Note: the full organizational structure includes other committees (e.g., data access and publication committee, DSMB, etc.)
Challenges Faced in Precision Promise

- Creating an Infrastructure: resources, vendor outsourcing/capabilities, governance and control
- Internal expertise: CMO, Business Development, Project Management, Strategic Alliance Management
- Determining the Trial Design: registration vs signal seeking goals
- Determining the adaptive features of design - statistical plan, simulations and modeling
- Regulatory strategy plan: FDA reviews and requests, IND, registration requirements
- Pharma engagement: provide time for learning curve to teach the design and get comfort level, developing the case for support, budget
- Organizational policies: data ownership, contracting
- Evolving field: medicine, technologies, statistics, genomics
- Maximizing efficiencies in speed: protocol finalization and other dependencies
What We’ve Learned

- Clearly defining governance structure and roles and responsibilities
- It will take longer than you think!
- Balancing the need to publicly announce for fundraising purposes versus creating false hope for patients about a trial that isn’t open and enrolling
- Cross-mingling of expert scientific teams will allow unique approaches to be tested in a rapid and efficient manner
- Need to push people beyond their comfort zones to learn why things work/don’t work in every single patient (biomarkers/biopsies)
- A new cooperative model that aligns key stakeholders (patients, families, researchers, clinicians, foundations, pharma, FDA) in a unique way to increase the pace and scale in which we can impact change
- A new national strategy to make significant advances in clinical trial options for pancreatic patients
- Smarter clinical trials embedded in strong preclinical data are in our future!
Summary: Why Master Protocols Why Now?

- Highly collaborative – integrates the best of knowledge of biology with agents and trial design
- Possible to ask and answer many questions
- Most of these trials reduce costs and time required for answers
- Offers opportunities for biomarker development within the trials – especially surrogate endpoints
- Coordinated screening and IRB review maximizes efficiencies
- Centralized Management ensures quality and reproducibility
- Advances (within and without the trial) can be integrated across the trial based on flexibility in the trial designs
- In adaptive setting, patients receive the agent best suited for their disease based on interval monitoring of trial data
- The ability to use shared controls saves time and money.
- These trials are learning systems
Q&A

Lisa LaVange
Gillings School of Global Public Health, University of North Carolina

Louis DeGennaro
The Leukemia & Lymphoma Society

Victoria Manax
Pancreatic Cancer Action Network

Anna Barker
School of Life Sciences, Arizona State University
MODERATOR
So You Want to Start a Master Protocol Trial…

Watch the recording
Using Innovative Financial and Business Models to Speed Science to Patients

February 20, 2019
1-2 pm ET
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