

# Adaptive Clinical Trial Design: Beyond Theory into Reality



Design-IT 2013

An INTERACTIVE Adaptive Clinical Trial Design Symposium

## Conference Brochure 2013

Bethesda North Marriott Hotel and Conference Center  
Bethesda, Maryland

Tuesday - Wednesday  
April 9 - 10, 2013

Sponsored by:





## Welcome

### Advancing Promising Trials Into Treatments

Have you attended a workshop on adaptive designs and been left with lingering questions about how you could actually move an idea for a clinical trial forward using some of these emerging methods? Have you wanted the opportunity for a personalized consultation with adaptive design experts? Adaptive designs for clinical trials are growing in popularity, although certain challenges exist to implementing them. The clinical and statistical teams must collaborate intensely to understand with good reasons, the advantages and disadvantages associated with potential adaptive design strategies. The goal is to design a trial that, regardless of the outcome, leaves the investigators with fewer regrets (i.e. if I had only looked at a three-hour treatment window instead of six) and leaves greater clarity for the scientific community – a clinical trial result that allows us to confidently either adopt the treatment or move on to the next most promising agent or strategy.

This unique *interactive* symposium will demonstrate how the process of planning an adaptive design trial has worked within the NIH- and FDA-funded cooperative project: Adaptive Designs Advancing Promising Trials Into Treatments (ADAPT-IT). In addition, we will show this interactive process in real time using examples pre-submitted by symposium attendees. Attendees will observe and participate in interactions between statisticians and trial clinicians, illustrating questions clinicians and statisticians should be asking each other during trial planning. One-on-one consultations between participants and course faculty will be included in the program. During the course of this two-day symposium, the selected trials will be simulated and potential results will be presented and discussed. Our team will work to advance your ideas and facilitate your decision making regarding what flexible adaptive elements might be best for your scientific questions.

#### Conference Objectives/Aims:

1. Introduce adaptive techniques that may enhance the efficiency and the potential value of clinical trials
2. Present examples of the evolution of an adaptive trial design based on scientific, statistical, and operational concerns
3. Participate in the interactive process of moving from trial idea to adaptive design concepts in real time
4. Illustrate the purpose and value of clinical trial simulation

#### Conference Faculty:

Faculty for this conference will include clinical trial and regulatory leaders with practical experience in the design and conduct of trials using these innovative designs.

William Meurer, MD, MS (Chair)  
University of Michigan

William Barsan, MD  
University of Michigan

Don Berry, PhD  
Berry Consultants

Scott Berry, PhD  
Berry Consultants

Kristin Broglio, MS  
Berry Consultants

Jason Connor, PhD  
Berry Consultants

Sarah Davis, MS  
UCSF

Michael Feters, MD, MHA, MA  
University of Michigan

Roger Lewis, MD, PhD  
Harbor- UCLA Medical Center

John Scott, PhD  
FDA/CBER

Robert Silbergleit, MD  
University of Michigan

#### Sponsorship:

This course is sponsored by the NIH-/FDA-funded ADAPT-IT project. This is a unique public/private partnership between Berry Consultants, an international leader in adaptive clinical trial design, and the Neurological Emergencies Treatment Trials Network. Funding support is through U01NS073476.

# Design-IT 2013: Adaptive Clinical Trial Design: Beyond Theory into Reality

## An Interactive Adaptive Clinical Trial Design Symposium

### SESSIONS and REGISTRATION

## Schedule

### Tuesday, April 9th

- 8:00-8:45 Registration/Breakfast (provided)  
8:45-9:00 Welcome/Introduction  
*William Barsan, MD*
- 9:00-9:30 An Introduction to Flexible Adaptive Designs  
*Roger Lewis, MD, PhD*
- 9:30-10:00 Adapting to Seizure Management: The ESETT trial  
*William Meurer, MD, MS / Jason Connor, PhD*
- 10:00-10:15 Break  
10:15-11:00 Regulatory Perspective on Adaptive Designs in the Confirmatory Phase  
*John Scott, PhD*
- 11:00-11:45 Operationalizing an Adaptive Design - A Data Center Perspective  
*Sarah Davis, MS*
- 11:45-1:00 Lunch (provided)  
1:00-1:15 Overview of Design Sessions  
*William Barsan, MD*
- 1:15-2:15 Design-IT Now Session A: Clinical Presentation and Q/A Session  
*Berry Consultants*
- 2:15-2:30 Break  
2:30-3:30 Design-IT Now Session B: Clinical Presentation and Q/A Session  
*Berry Consultants*
- 3:30-3:45 Overview of Breakout Sessions and Description of Day 2  
3:45-5:15 Design-IT Now Session C: Small Groups

Adaptive design consultations. Four teams consisting of a clinician/biostatistician will be available to meet with researchers who submitted ideas or just have general ideas. Potential adaptive solutions to trial design issues will be proposed. One of the ideas pitched from this session will be selected by the course faculty and will be presented the next day.

### Wednesday, April 10th

- 8:00-8:30 Breakfast (provided)/Networking  
8:30-9:00 Understanding Simulations and Their Value in Clinical Trial Planning  
*William Meurer, MD, MS / Scott Berry, PhD*
- 9:00-10:00 Concept/Performance Presentation: Design-IT Now Session A  
10:00-11:00 Concept/Performance Presentation: Design-IT Now Session B  
11:00-11:15 Break  
11:15-12:15 Concept/Performance Presentation: Design-IT Now Session C  
12:15-1:00 Lunch (provided)  
1:00-1:30 Insights from the Mixed Methods Evaluation of the ADAPT-IT Project  
*Michael Fetters, MD, MHA, MA*
- 1:30-2:00 Vote on "Best Design"  
*William Barsan, MD*
- 2:00-2:30 Discussion of Next Steps to Move Forward / Adjourn  
*Roger Lewis, MD, PhD*

## Registration & Housing

### Registration:

We hope you will join us for this unique educational experience. Registration is FREE, but is limited to 100 people.

To REGISTER please click on this link:

[http://nett.umich.edu/nett/design\\_it\\_conference\\_registration](http://nett.umich.edu/nett/design_it_conference_registration)

The DEADLINE for reservations is:  
**MONDAY, MARCH 18, 2013.**

You will receive a confirmation email once your registration has been entered.

### Housing:

The meeting will be held at the beautiful and convenient Bethesda North Marriott Hotel and Conference Center:

5701 Marinelli Road  
North Bethesda, MD 20852

A block of rooms is being held for meeting attendees at a special rate of \$199.00/night. The special rate is guaranteed for reservations made on or before March 18, 2013. Attendees are responsible for making their own reservations and for the cost of their hotel rooms.

Reservations can be made by phoning Marriott Reservations at 1-877-212-5752 and mentioning the meeting name and number (ADAPT-IT Educational Symposium M-0509JB). You may also go directly to this conference specific on-line group reservation link:

<https://resweb.passkey.com/go/7cbf0462>

The following link to the hotel website will provide you with information regarding the facilities and amenities offered by this venue.

<http://www.marriott.com/hotels/travel/was-bn-bethesda-north-marriott-hotel-and-conference-center/>

# Design-IT 2013: Adaptive Clinical Trial Design: Beyond Theory into Reality

## An INTERACTIVE Adaptive Clinical Trial Design Symposium

**INTERACTIVE. UNIQUE. INNOVATIVE.**

## Submit Your Trial Idea

This is an INTERACTIVE symposium and relies on the active participation of its attendees to be successful. As part of the symposium, we will feature several clinical trial ideas submitted by attendees prior to the meeting. The Design-IT faculty will select two of the submitted ideas for presentation on Day One of the conference. The faculty will actively engage with the team to develop possible solutions to design issues. You will need to provide us with a description of the disease/injury process that your trial will focus on, the treatments you propose to study, goals of the trial, major unknowns relevant to the trial design, and any interesting features that the trial may have. (See the example to the right.) **Detailed information on how to format and submit your design ideas will be sent to you by email as soon as you have registered.**

## Do You Have Questions?

### General Questions:

Shirley Frederiksen, RN, MS  
[sfred@umich.edu](mailto:sfred@umich.edu)

### Registration Details:

Jeff Clevenger, AB, AM  
[jdclev@umich.edu](mailto:jdclev@umich.edu)

### Submitting Trial Ideas:

William Meurer, MD, MS  
[wmeurer@umich.edu](mailto:wmeurer@umich.edu)

## Example

### Describe the Disease/Injury Process your trial focuses on:

Spinal cord injury has devastating consequences and no currently effective neuroprotective treatments.

### Please describe the treatment(s) you would study:

Hypothermia versus enforced normothermia for acute treatment of cervical spinal cord injury.

### Describe the possible goals of the trial:

To determine the optimum duration of hypothermia and to determine whether hypothermia is more effective than temperature control (enforced normothermia).

### Describe major unknowns in this specific area that are relevant to trial design:

Hypothermia is a promising treatment for spinal cord injury based on smaller animal models and early clinical experience. The optimal duration of cooling in humans is not currently well known. In addition, since hypothermia has risks, it is important to understand whether it actually works using a randomized controlled trial design.

### Describe any interesting features of the trial:

We would like to balance feasibility with having a shorter enrollment window (say, 4-6 hours), knowing that many treatments for acute neurological recovery have failed because of the use of windows that are too long. Many patients are transferred to trauma centers, and some patients may need to go to the OR early, so we want to ensure that we can get as many patients as early as possible to maximize the chance of success.