Subject: ENAR 2024 Invited Session Proposal Form
Date: Tuesday, June 20, 2023 at 1:34:12 PM Eastern Daylight Time
From: Google Forms
To: Carly Lupton Brantner

Google Forms

Thanks for filling out ENAR 2024 Invited Session Proposal Form

Here's what was received.

Edit response

ENAR 2024 Invited Session Proposal Form

All invited sessions are scheduled for 105 minutes. We will consider different formats including a session with 4 speakers, a session with 3 speakers plus a discussant, or a panel discussion. Each participant may be a speaker/panelist in at most one invited or contributed session.

All session proposals will be evaluated for acceptance using the ENAR Invited Session Abstract Review Rubric, which reflects the meeting theme "ENAR – A Home for Every Biostatistician" and ENAR's continued commitment to putting together a high-quality scientific program that best serves the needs of all ENAR members.

Please contact the Program Chair, Sameera Wijayawardana, at wijayawardanasr@lilly.com or Co Chair, Christina Mehta at christina.mehta@emory.edu for any queries.

Email *

clupton1@jhu.edu

Session Title *

Integrating Data from Multiple Sources to Estimate Causal Effects

Session Motivation *

Reliable estimation of causal effects is vital in order to make informed treatment decisions. There has been a burgeoning interest in utilizing multiple data sources to improve causal effect estimation throughout the causal inference literature. Combining datasets has strong potential to improve the estimation and generalizability of causal effects; however, there are many intricacies that must be effectively accounted for to ensure accurate estimation. For example, combining randomized controlled trials (RCTs) together can improve the power for identifying treatment effect moderation, but heterogeneity across trials can complicate this assessment. Similarly, bringing in observational data can effectively supplement trial data, but the observational and trial data should be sufficiently comparable and population differences need to be adjusted for. This session will include three talks that explore approaches for integrating data from different sources to estimate causal effects. We will reflect on the benefits and challenges that come with combining data and explain methods that researchers can use to best leverage multiple datasets. Two of the presentations focus on combining RCTs to estimate heterogeneous treatment effects, while the third explores using real world data to supplement an RCT and construct a hybrid control arm. Finally, the session will include a discussant with expertise in this area of data integration, specifically surrounding the combination of observational and trial data and the importance of taking into account the level of overlap in the two populations. We feel that this session would bring significant value to ENAR attendees looking to learn more about causal inference and the role that data integration can play in effectively estimating treatment effects.

Please select the category that best aligns with your invited session proposal. *

Theory/Methodology Development

ENAR -- A Home for Every Biostatistician is our 2024 theme. Please check which of these aspects of diversity and inclusion are reflected in your proposal. Check all

that apply.
*

- [x] Range of Degrees
- [x] Speakers Years of Experience (Junior/Assistant, Mid-career, Senior)
- [x] Gender
- [x] Race/Ethnicity
- [x] Sexual Orientation
- [x] Organization (Academia, Industry, Government)
- [ ] Other: .....

Session Organizer

(* required; to be filled by the Organizer)

First Name *

Carly

Last Name *

Brantner

Affiliation *

Johns Hopkins University

Email *

clupton1@jhu.edu

Is this an IMS Invited Session? *

- ○ YES
- ◉ NO

Other pertinent contact information

Session Chair

Please note that per ENAR guidelines, a person cannot hold multiple roles within one session (e.g., Chair and present a paper in the same invited session). Additional information on participant opportunities and limits can be found on the ENAR website.

First Name *

Trang

Last Name *

Nguyen

Affiliation *

Johns Hopkins University

Email *

trang.nguyen@jhu.edu

Other pertinent contact information

Speaker #1

First Name *

Carly

Last Name *

Brantner

Please confirm that this individual has agreed to participate and this is the only ENAR 2024 invited session proposal for which this individual has agreed to participate. *

☑ Yes, I confirm

Affiliation *

Johns Hopkins University

Email *

clupton1@jhu.edu

Talk Title *

Combining Trials to Estimate Heterogeneous Treatment Effects in a Target Sample

Abstract for Speaker #1 (Length: 1200 character max) *

Clinicians are often motivated to determine which treatment would work best for an individual based on their observed characteristics, but reliably doing so requires large amounts of data and adjustment for confounders. One approach to estimate these effects can be through combining randomized controlled trials (RCTs). However, methods that combine RCTs often yield estimates that are conditional on trial membership, so applying these models to a target sample is not straightforward. This presentation introduces approaches for estimating conditional average treatment effects (CATEs) for a target sample, based on a model derived from multiple RCTs. The approaches draw from meta-analytic prediction intervals to create 95% intervals for the CATEs in the target sample. We examine simulations based on real data that compare meta-analytic prediction intervals with resampling-based intervals from causal forests. We discuss the trade-offs of parametric and non-parametric approaches and how researchers might choose between them. These approaches allow future researchers to effectively leverage multiple RCTs to estimate treatment effects in a target sample of interest.

Does Speaker #1 have any time conflicts? *

- [ ] Yes
- [x] No

If Speaker #1 has any time conflicts, please specify these below.

Speaker #2

First Name *

Benjamin

Last Name *

Ackerman

Please confirm that this individual has agreed to participate and this is the only ENAR 2024 invited session proposal for which this individual has agreed to participate. *

☑ Yes, I confirm

Affiliation *

Janssen Research & Development

Email *

ben.a.ackerman@gmail.com

Talk Title *

Augmenting Randomized Trials With Real-World Data: A Simulation Study Evaluating Methods For Hybrid Control Arm Analyses

Abstract for Speaker #2 (Length: 1200 character max) *

Randomized trials (RCTs) are considered the gold standard for estimating causal effects of new therapeutics, yet statistical challenges remain in detecting effects in rare disease populations. Innovative approaches exist to supplement trials with real-world data (RWD) by constructing a hybrid control arm to increase study sample size and power. RWD suitability for this use case is critical and dependent on RCT design, study population and outcome measure. Even with high-quality RWD, differences in study populations must be properly accounted for and eligibility criteria must be aligned to ensure comparability, particularly for characteristics that are prognostic of the outcome. We present propensity score weighting and frequentist borrowing methods to align study populations when conducting hybrid control arm analyses. We simulate studies under varying degrees of covariate imbalance and illustrate conditions where use of these RCT-augmentation methods yield greater precision than RCT-only analyses while maintaining the type-1 error. We present criteria when selecting suitable RWD, and highlight considerations when implementing the proposed methods for hybrid control arm analyses.

Does Speaker #2 have any time conflicts? *

- [ ] Yes
- [x] No

If Speaker #2 has any time conflicts, please specify these below

Speaker #3

First Name *

Cathy

Last Name *

Shyr

Please confirm that this individual has agreed to participate and this is the only ENAR 2024 invited session proposal for which this individual has agreed to participate. *

☑ Yes, I confirm

Affiliation *

Vanderbilt University Medical Center

Email *

cathy.shyr@vumc.org

Talk Title *

Multi-study R-learner for Heterogeneous Treatment Effect Estimation

Abstract for Speaker #3 (Length: 1200 character max) *

Estimating heterogeneous treatment effects is crucial for precision medicine. While multiple studies can improve the generalizability of results, leveraging them for estimation is statistically challenging. Existing methods assume identical treatment effects across studies, but this may be violated due to various sources of between-study heterogeneity. To this end, we propose a unifying framework for heterogeneous treatment effect estimation robust to between-study heterogeneity in the nuisance functions and treatment effects. Our approach,

the multi-study R-learner, extends the R-learner to obtain principled statistical estimation with machine learning (ML) in the multi-study setting. It incorporates ML for estimating heterogeneous treatment effects, nuisance functions, and membership probabilities, which borrow strength across studies. Our method achieves robustness in confounding adjustment through its loss function and can leverage both randomized and observational studies. We provide asymptotic guarantees in the case of series estimation and illustrate using cancer data that it has the lowest error compared to existing methods in the presence of between-study heterogeneity.

Does Speaker #3 have any time conflicts? *

- [ ] Yes
- [x] No

If Speaker #3 has any time conflicts, please specify these below

Discussant (or Speaker #4)

First Name

Irina

Last Name

Degtiar

Please confirm that this individual has agreed to participate and this is the only ENAR 2024 invited session proposal for which this individual has agreed to participate.

☑ Yes, I confirm

Affiliation

Mathematica Policy Research

Email

IDegtiar@mathematica-mpr.com

This individual is a:

☑ Discussant

☐ Speaker

Talk Title for Speaker #4 (if applicable). *If Discussant, please write Discussant in this section.

Discussant

Abstract for Speaker #4 (Length: 1200 character max) *If Discussant, please write Discussant in this section.

Discussant

Does Discussant or Speaker #4 have any time conflicts?

☐ Yes

- [x] No

Additional Information

Choice of potential session sponsors does not affect the chance that your session is selected. It is for the benefit of ENAR attendees to select sessions of specific areas.

Potential session sponsors (pick up to 3) *

- [x] ENAR
- [ ] IMS
- [ ] JEDI (Justice, Equity, Diversity, Inclusion) Outreach Group
- [ ] ASA Committee on Minorities in Statistics
- [ ] ASA Section: Biometrics
- [ ] ASA Section: Biopharmaceutical
- [ ] ASA Section: Statistical Consulting
- [x] ASA Section: Statistics in Epidemiology
- [ ] ASA Section: Teaching Statistics in Health Sciences

Additional information you'd like to share with the Program Committee (e.g., scientific quality; timeliness; clarity and cohesiveness of the talks; speaker diversity; broad application and/or major advancement to a field).

This topic is of high interest in the causal inference literature, with many researchers working to leverage multiple datasets to improve causal effect estimation. We feel that this session would generate interest from individuals working all across academia, industry, and government. Our speakers are a diverse group with strong expertise in this field of causal inference. Thank you for your consideration of this session!

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