DIVISION OF STATISTICS
NORTHERN ILLINOIS UNIVERSITY
Colloquium

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TITLE: A Systematic Scoring Procedure for Subgroup Identification from Randomized Controlled Trials

DATE: Friday March 4, 2016
TIME: 2:00 p.m.
PLACE: DUSABLE HALL 212

Abstract
To evaluate a new therapy versus a control via a randomized, comparative clinical study or a series of trials, due to heterogeneity of the study patient population, a pre-specified, predictive enrichment procedure may be implemented to identify an enrichable subpopulation. For patients in this subpopulation, the therapy is expected to have a desirable overall risk-benefit profile. To develop and validate such a “therapy-diagnostic co-development” strategy, a three-step procedure may be conducted with three independent data sets from a series of similar studies or a single trial. At the first stage, we create various candidate scoring systems based on the baseline information of the patients via, for example, parametric models using the first data set. Each individual score reflects an anticipated average treatment difference for future patients who share similar baseline profiles. A large score indicates that these patients tend to benefit from the new therapy. At the second step, a potentially promising, enrichable subgroup is identified using the totality of evidence from these scoring systems. At the final stage, we validate such a selection via two-sample inference procedures for assessing the treatment effectiveness statistically and clinically with the third data set, the so-called holdout sample. When the study size is not large, one may combine the first two steps using a “cross-training-evaluation” process. The entire enrichment procedure is illustrated with the data from a cardiovascular trial to evaluate a beta-blocker versus a placebo for treating chronic heart failure patients.