

Statistical Methods for Causal Inference in Observational Studies

<https://www.neurodegenerationresearch.eu/survey/statistical-methods-for-causal-inference-in-observational-studies/>

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Country

USA

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Statistical Methods for Causal Inference in Observational Studies

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1

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Amyotrophic Lateral Sclerosis, Gastrostomy, Observational Study, Statistical Methods, Registries

Research Abstract

? DESCRIPTION (provided by applicant): The goal of this project is to develop innovative statistical methods for causal inference in observational studies that can handle time-varying confounders, censoring by death, and missing data in addition to selection bias, and to answer important clinical questions on management of the Amyotrophic Lateral Sclerosis (ALS) disease. The existing studies on addressing these questions have several limitations including

their small to moderate sample sizes and the use of limited clinical data and statistical methods that did not adequately address complicating issues including selection bias commonly encountered in observational studies. This study will use the data from the Emory ALS Registry with several notable strengths including a large sample size of over 1,700 patients with long-term follow-ups and collection of extensive clinical information at each clinic visit since 1997. As a result, the Emory ALS Registry is uniquely suited for answering important clinical questions. The analysis of the ALS Registry presents several challenges including time-varying confounders, censoring by death, and missing data. The existing statistical methods cannot be applied directly to address all these issues. These considerations lead to three specific aims: 1) develop a new propensity score for balancing time-varying covariates in observational studies, the propensity process, and develop statistical methods for estimating causal effects based on the propensity process; 2) develop statistical methods for assessing the survivor average causal effects (SACE) in the presence of missing data in observational studies with time-varying covariates; and 3) perform systematic evaluation of the proposed methods in Aims 1 and 2 through extensive simulations and perform analysis of the Emory ALS Registry data. Progress in all aims will be guided by and evaluated on the Emory ALS Registry, and by extensive simulation studies. The proposed methods will enable us to answer important clinical questions, e.g., assessing the effects of procedures including the percutaneous endoscopic gastrostomy (PEG) and the non-invasive positive pressure ventilation (NIPPV) on patient outcomes. The proposed methods are general and promise similar benefits to a wide range of observational studies and registries, since similar data structures and analytical issues are often encountered in these types of studies.

Further information available at:

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