Targeted ML Solutions

Selected Publications and Research

Selected Case Studies in Health Care

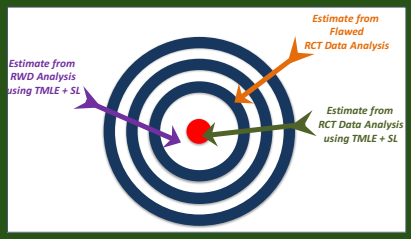
Drug safety and effectiveness evaluations using TMLE

Regulatory Collaborations

Research Papers co-authored with colleagues at the US Food and Drug Administration

FDA & Industry Perspectives

ASA Biopharmaceutical Real-World Evidence Working Group collaborations



Targeted Machine Learning

The new standard for generating reliable evidence from data

Targeted Learning: Selected Case Studies in Healthcare

1. Efficacy and safety of Belumosudil as Compared with Best Available Therapy for the Treatment of cGVHD in the United States. *Blood Advances* (2026). [access]

2. Sustained glucagon-like peptide-1 receptor agonist treatment improves glycemic control and reduces all-cause mortality compared to dipeptidyl peptidase-4 inhibitors: A real-world target trial emulation in type 2 diabetes. *Diabetes, Obesity and Metabolism* (2026). [access]

3. Intravenous amino acid supplementation reduces 28-day mortality in sepsis: a retrospective cohort study from MIMIC-IV database and Mendelian randomization analysis. *British Journal of Nutrition* (2026). [access]

4. Lower rate of ischemic events with alirocumab in patients with atherosclerotic cardiovascular disease but without prior acute ischemic events. *European Heart Journal* (2025). [access]

5. GLP-1RA comparative effectiveness against dementia onset relative to other antidiabetic medications in a large, multi-site cohort of patients with type 2 diabetes. *Alzheimer's and Dementia* (2025). [access]

6. Covid-19 Vaccination Timing, Relative to Acute COVID-19, and Subsequent Risk of Long COVID. *medRxiv preprint* (2025). [access]

7. Comparative Mortality Risk of Aripiprazole, Olanzapine, Quetiapine and Risperidone in Alzheimer's Disease: A Real World Cohort Study with Treatment Effect Heterogeneity Analysis. *medRxiv preprint* (2025). [access]

8. Medical Cannabis Use and Healthcare Utilization Among Patients with Chronic Pain: A Causal Inference Analysis Using TMLE. *Pharmacy* (2025). [access]

9. Glucose-Lowering Medication Classes and Cardiovascular Outcomes in Patients With Type 2 Diabetes. *JAMA Network Open* (2025). [access]

10. A targeted likelihood estimation comparing cefepime and piperacillin/tazobactam in critically ill patients with community-acquired pneumonia (CAP). *Scientific Reports* (2024). [access]

11. Statistical methods to control for confounders in rare disease settings that use external control. *Journal of Biopharmaceutical Statistics* (2024). [access]

12. SSRI Use During Acute COVID-19 Infection Associated with Lower Risk of Long COVID Among Patients with Depression. *medRxiv preprint* (2024). [access]

Targeted Learning: Selected Regulatory Collaborations

1. Targeted Learning: Toward a future Informed by Real-World Evidence, *Statistics in Biopharmaceutical Research* (2024). [access]
2. Evaluating and Improving Real-World Evidence with Targeted Learning, *BMC Medical Research Methodology (2023)*. [access]
3. Developing a Targeted Learning-Based Statistical Analysis Plan, *Statistics in Biopharmaceutical Research (2023)*. [access]
4. A practical guide to specifying a super learner. *International Journal of Epidemiology* (2023). [access]

Targeted Learning: Selected FDA and Industry Perspectives

1. Use of Real-World Data and Real-World Evidence in Rare Disease Drug Development: A Statistical Perspective. *Clinical Pharmaceutical and Therapeutics* (2025). [access]
2. Challenges and Possible Strategies to Address Them in Rare Disease Drug Development: A Statistical Perspective. *Clinical Pharmaceutical and Therapeutics* (2025). [access]
3. Targeted Learning for Improved Causal Inference in Randomized Controlled Trials. *ASA Biopharmaceutical Report* (2024). [access]
4. Sensitivity Analysis for Unmeasured Confounding in Medical Product Development and Evaluation using Real-World Evidence. *Statistics in Biopharmaceutical Research* (2024). [access]
5. Examples of Applying RWE Causal-Inference Roadmap to Clinical Studies. *Statistics in Biopharmaceutical Research* (2023). [access]
6. The Current Landscape in Biostatistics of Real-World Data and Evidence: Causal Inference Frameworks for Study Design and Analysis. *Statistics in Biopharmaceutical Research* (2023). [access]
7. Biostatistical Considerations When Using RWD and RWE in Clinical Studies for Regulatory Purposes: A Landscape Assessment. *Statistics in Biopharmaceutical Research* (2023). [access]

Targeted Machine Learning: Introductory Materials

1. Why We Need a Statistical Revolution. *Sense about Science, USA (2015)*. [access]
2. A Causal Roadmap for Generating High Quality Real-World Evidence. *Journal of Clinical and Translational Science (2023)*. [access]
3. An Application of the Targeted Learning Roadmap in Two Safety Monitoring Case Studies: Causal inference and outcome prediction using electronic health records data. *Journal of Clinical and Translational Science (2023)*. [access]
4. Commentary on ``Nonparametric identification is not enough, but randomized controlled trials are'': Statistical considerations for generating reliable evidence across a spectrum of studies that increasingly involve real-world elements. *Observational Studies* (2025). [access]
5. Targeted Learning Webinar Series on YouTube: A series of 16 lectures by various experts in the field. [access]