Assessing External Validity Over Worst-case Subpopulations

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Based on a joint work with Sookyo Jeong <u>https://arxiv.org/abs/2007.02411</u>

Potential outcomes

- A feature vector $X \in \mathbb{R}^k$
- Potential outcomes: Y(1), Y(0)

Average Treatment Effect (ATE) $ATE = \mathbb{E}[Y(1) - Y(0)]$ $= \mathbb{E}_{X \sim P_{X}} \left[\mathbb{E}[Y(1) | X] - \mathbb{E}[Y(0) | X] \right]$

• P_X is the data generating distribution for X

- A treatment assignment $Z \in \{0,1\}$
- Observe Y := Y(Z), never Y(1 Z)





• Demographic compositions shift over time

Change in share from 2009



What if *P_X* changes?

What if P_X changes?



[Tipton et al. 2019] The convenience of large urban school districts: a study of recruitment practices in 37 randomized trials

Even for carefully designed randomized trials, "statistics" starts only at treatment assignment, with big biases in selection into study

Distribution of log-district size in studies versus total population

- "Clinical trials for new drugs skew heavily white" [Oh et al. '15, Burchard et a
 - than 5% of participants were non-white
- Especially problematic when treatment effect is heterogeneous

[Leigh et al. '16, Imai et al. '13, Gijsberts et al. '15, Basu et al. '17, Baum et al. '17, Duan et al. '19]



- Out of 10,000+ cancer trials, less than 2% focused on racial minorities, and less

• Recently, two large trials with n = 5K-10K had opposite findings on a treatment to lower blood pressure on cardiovascular disease [ACCORD '10, SPRINT '15]



Potential solution?

 Directly estimate conditional average treatment affect (CATE) using ML methods?

[Leigh et al. '16, Imai et al. '13, Gijsberts et al. '15, Basu et al. '17, Baum et al. '17, Duan et al. '19, Nie and Wager '20]

- ML models perform very poorly on underrepresented groups
- ML estimates are unstable and resulting inference is underpowered
- Predefined subgroup analysis difficult due to intersectionality

Effect of Medicaid enrollment on doctor's office utilization



Effect size









Automatically find worst-off subpopulations and measure treatment effect on them





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Worst-case subpopulation

Notation $Q_X \succeq \alpha \quad \longleftrightarrow \quad \left\{ Q_X : \frac{\exists \text{probability } Q'_X, \text{ and } a \ge \alpha}{\text{s.t. } P_X = aQ_X + (1-a)Q'_X} \right\}$

subpopulation with proportion larger than $\alpha \in (0, 1]$

worst-case treatment over subpopulation larger than $\alpha \in (0, 1]$

$$WTE_{\alpha} := \sup_{Q_X \succeq \alpha} \mathbb{E}_Q$$

where $\mu^{\star}(X) := \mathbb{E}[Y(1) - Y(0) \mid X]$ is the conditional average

treatment effect (CATE).

Recap

- Covariates: X
- Treatment assignment: Z
- Potential outcome: Y(0), Y(1)
- Response Y := Y(Z)

- $\mu^{\star}(X)$



Sensitivity analysis

- Posit a set of "plausible" changes to P_X , and take worst-case over them
- If effects are still valid under plausible violations, we can certify robustness
- Sensitivity of a finding: magnitude of violation when endpoint crosses a threshold
- Today: Worst-case bounds on the Doubly Robust / AIPW estimator



$${}^{\mathsf{nd}} WTE_{\alpha} := \sup_{Q_X \succeq \alpha} \mathbb{E}_{Q_X} [\mu^*(X)]$$

Sensitivity analysis



Sensitivity analysis

- Does not assume a fixed target; often appropriate for operational decisions
- Heuristically, set α small if the collected data is not diverse
- Conservative but can still be useful; future work needed on this
- Need to be accompanied by a design-based perspective to maximizing diversity in ${\cal P}_{{\cal X}}$

- Evaluate effect of Medicaid enrollment on doctors' office utilization
- Medicaid costs \$553 billion/yr; need to ensure valid effects through time
- Outcome: visit to doctors in the two-weeks prior to a random survey date
- Control for demographics, medical history, employment, earnings, insurance, government assistance etc (d = 396)
- Take the viewpoint of an analyst in 2009 (n = 82,993)



Evaluate effect of Medicaid enrollment on doctors' office utilization in 2009



Evaluate effect of effect of Medicaid enrollment on doctors' office utilization

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Welfare attitudes experiment

- Evaluate effect of wording on sur the poor")
- WTE guarantees positive findings even for small subpopulations
- WTE is stable across model classes used, similar to ATE, unlike CATE



• Evaluate effect of wording on survey results ("welfare" vs "assistance to



TA. SIID Lemma (Shapiro et al. '09) $\sup \mathbb{E}_{Q_X}[\mu^{\star}(X)] = \mathbb{E}[\mu^{\star}(X)h^{\star}(X)]$ $Q_X \succeq \alpha$ where $h^{\star}(x) := \frac{1}{\alpha} \mathbf{1} \left\{ \mu^{\star}(x) \ge P_{1-\alpha}^{-1}(\mu^{\star}) \right\}$



Estimation Approach

• Use ML methods to fit nuisance parameters

$$\mu_z^{\star}(X) = \mathbb{E}[Y(z) \mid X = x], \ z$$

$$e^{\star}(X) = \mathbb{P}(Z = 1 \mid X)$$

- Design an mean zero augmentation term that includes nuisance parameters

$$WTE_{\alpha} + \mathbb{E}\left[h^{\star}(X)\left(\frac{Z}{e^{\star}(X)}(Y - \mu_{1}^{\star}(X)) - \frac{1 - Z}{1 - e^{\star}(X)}(Y - \mu_{0}^{\star}(X))\right)\right]$$

Neyman orthogonal: Directional derivative w.r.t. nuisance parameters, taken at the true nuisance value $(\mu_1^{\star}, \mu_0^{\star}, e^{\star}, h^{\star})$ is zero. [Neyman '59, Chernozhukov et al. '18]

 $z \in \{0, 1\}$

Recap

- Covariates: X
- Treatment assignment: Z
- Potential outcome: Y(0), Y(1)

$$h^{\star}(X) = \frac{1}{\alpha} \mathbf{1} \left\{ \mu^{\star}(X) \ge P_{1-\alpha}^{-1}(\mu^{\star}) \right\}$$

Today: Construct a WTE estimator insensitive to error in nuisance estimates



Assumptions

Standard; required for identification and estimation of ATE

- No unobserved confounding: $Y(0), Y(1) \perp Z \mid X$
- Overlap: $\exists c > 0 \ s.t. \ \mathbb{P}(e^{\star}(X) \in [c, 1 c]) = 1$
- SUTVA: single version of treatment, no interference between units

Recap

- Covariate X, Treatment Z
- Potential outcome: Y(0), Y(1)
- Propensity score $e^{\star}(X) = \mathbb{P}(Z = 1 | X)$



Main Results

Theorem (Jeong & N. '20)

2. σ_{α}^2 is the optimal asymptotic variance

- and observational studies

Under slower-than-parametric rates of convergence on the nuisance parameters, $\sqrt{n}(\hat{w}_{\alpha} - WTE_{\alpha}) \Rightarrow N(0,\sigma_{\alpha}^2)$

Central limit rates even when nuisance estimates converge more slowly

• Augmented estimator is semiparametrically efficient for both randomized



- Worst-case bounds on the Doubly Robust / AIPW estimator under distribution shift
- Allow flexible use of ML methods to estimate nuisance parameters
- Central limit results even when nuisance parameters converge slower
- Our procedures are optimal; semiparametrically efficient

Summary



https://arxiv.org/abs/2007.02411

