

# Randomized Experiment

Causal Inference using Machine Learning  
Master in Economics, UNT

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# The first RCT

"Let us divide them in halves, let us cast lots, that one half of them may fall to my share, and the other to yours; I will cure them without bloodletting and sensible evacuation; but do you do as ye know [...] we shall see how many Funerals both of us shall have."

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- **Peirce (1885)**: Used random sequencing in psychology to prevent bias from expectations, anticipating randomization principles.
- **Gossett and Fisher (1920s)**: Gossett mentioned random plot placement; Fisher formalized randomization as essential for causal inference.

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- Proposed an estimator for the Variance of the Average Treatment Effect (ATE) in randomized experiments.

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- Emphasized the need for physical randomization to eliminate confounding variables.
- Proposed methods for testing hypotheses in a controlled experimental setup.



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- Fisher introduced significance testing and p-values for general hypothesis, while Neyman was more concerned with unbiased estimation of ATE.
- Together, they laid the groundwork for randomized experiments and causal inference.

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- For two treatment groups,  $D$  is a binary vector with  $2^N$  possible values.



# Assignment Mechanism

**Assignment Mechanism:** Given a population of  $N$  units, the assignment mechanism is a row-exchangeable function, denoted as  $\Pr(D|X, Y(0), Y(1))$ , which takes values in the interval  $[0, 1]$  and satisfies:

$$\sum_{D \in \{0,1\}^N} \Pr(D|X, Y(0), Y(1)) = 1$$

for all possible values of  $X$  (covariates),  $Y(0)$ , and  $Y(1)$  (potential outcomes). (Row-exchangeability implies that the order of units within vectors or matrices is irrelevant to the function  $\Pr(\cdot)$ .)

## Example: Assignment Mechanism with Two Units

Define the **treatment effect** for unit  $i$  as:  $\tau_i = Y_i(1) - Y_i(0)$

$$\Pr(D|X, Y(0), Y(1)) = \begin{cases} 1 & \text{if } \tau_2 > \tau_1 \text{ and } D = \begin{bmatrix} 0 \\ 1 \end{bmatrix} \\ 1 & \text{if } \tau_2 < \tau_1 \text{ and } D = \begin{bmatrix} 1 \\ 0 \end{bmatrix} \\ \frac{1}{2} & \text{if } \tau_2 = \tau_1 \text{ and } D \in \left\{ \begin{bmatrix} 0 \\ 1 \end{bmatrix}, \begin{bmatrix} 1 \\ 0 \end{bmatrix} \right\} \\ 0 & \text{if } D \in \left\{ \begin{bmatrix} 0 \\ 0 \end{bmatrix}, \begin{bmatrix} 1 \\ 1 \end{bmatrix} \right\} \\ 0 & \text{if } \tau_2 < \tau_1 \text{ and } D = \begin{bmatrix} 0 \\ 1 \end{bmatrix} \\ 0 & \text{if } \tau_2 > \tau_1 \text{ and } D = \begin{bmatrix} 1 \\ 0 \end{bmatrix} \end{cases}$$

# Unit Assignment Probability

The **unit-level assignment probability** for unit  $i$  is defined as:

$$p_i(X, Y(0), Y(1)) = \sum_{D: D_i=1} \Pr(D|X, Y(0), Y(1)),$$

# Propensity Score

The **propensity score** at  $x$  is the average unit assignment probability for units with  $X_i = x$ . It is defined as:

$$e(x) = \frac{1}{N(x)} \sum_{i: X_i = x} p_i(X, Y(0), Y(1)),$$

# Example: Propensity Score

1.  $D = (0, 0, 0, 0)$   $P(D = 1) = 0$
2.  $D = (1, 0, 0, 0)$   $P(D = 2) = \frac{3}{16}$
3.  $D = (0, 1, 0, 0)$   $P(D = 3) = \frac{2}{16}$
4.  $D = (0, 0, 1, 0)$   $P(D = 4) = 0$
5.  $D = (0, 0, 0, 1)$   $P(D = 5) = 0$
6.  $D = (1, 1, 0, 0)$   $P(D = 6) = \frac{1}{16}$
7.  $D = (0, 1, 1, 0)$   $P(D = 7) = \frac{2}{16}$
8.  $D = (0, 0, 1, 1)$   $P(D = 8) = \frac{1}{16}$

9.  $D = (1, 0, 1, 0)$   $P(D = 9) = \frac{3}{16}$
10.  $D = (1, 0, 0, 1)$   $P(D = 10) = \frac{2}{16}$
11.  $D = (0, 1, 0, 1)$   $P(D = 11) = \frac{2}{16}$
12.  $D = (1, 1, 1, 0)$   $P(D = 12) = 0$
13.  $D = (1, 0, 1, 1)$   $P(D = 13) = 0$
14.  $D = (0, 1, 1, 1)$   $P(D = 14) = 0$
15.  $D = (1, 1, 0, 1)$   $P(D = 15) = 0$
16.  $D = (1, 1, 1, 1)$   $P(D = 16) = 0$

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# Individualistic Assignment

**Definition 3.4 (Individualistic Assignment):** For some function  $q(\cdot) \in [0, 1]$ :

$$p_i(X, Y(0), Y(1)) = q(X_i, Y_i(0), Y_i(1)), \quad \text{for all } i = 1, \dots, N,$$

and

$$\Pr(D|X, Y(0), Y(1)) = c \cdot \prod_{i=1}^N q(X_i, Y_i(0), Y_i(1))^{D_i} (1 - q(X_i, Y_i(0), Y_i(1)))^{1-D_i}.$$

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- The constant  $c$  ensures that the probabilities sum to unity.

**Homework:** Compute the value of  $c$  for a generic assignment mechanism with two units and a binary treatment.

# Probabilistic Assignment Mechanism

**Probabilistic Assignment Mechanism:** Under this mechanism, each unit has a non-zero probability of being assigned to either treatment or control, ensuring randomness in the assignment process.

$$0 < \Pr(D_i = 1 | X, Y(0), Y(1)) < 1 \quad \text{for all units } i$$

# Unconfounded Assignment Mechanism

**Unconfounded Assignment Mechanism:** This mechanism assumes that assignment to treatment is independent of the potential outcomes, given the covariates. In other words, the assignment is “as good as random” conditional on covariates.

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$$\Pr(D|X, Y(0), Y(1)) = \Pr(D|X)$$

- Given individualistic assignment and unconfoundedness

$$\Pr(D|X, Y(0), Y(1)) = c \cdot \prod_{i=1}^N q(X_i)^{D_i} \cdot (1 - q(X_i))^{1-D_i}$$

so that

$$e(x) = q(x)$$

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# Randomized Experiment

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A classical randomized experiment is a randomized experiment with an assignment mechanism that is:

- (i) **Individualistic**: Each unit's treatment assignment depends only on its own covariates and potential outcomes, independent of other units.
- (ii) **Unconfounded**: Assignment to treatment is independent of potential outcomes given covariates, meaning assignment is “as good as random” conditional on covariates.

A **Bernoulli trial** is a classical randomized experiment where each unit is independently assigned to treatment or control, often based on a coin toss.

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- Each unit has a probability  $q$  of being assigned to treatment and  $1 - q$  of being assigned to control.
- Each unit's assignment is independent of others, meaning the assignment for one unit does not affect the assignment for another.
- The assignment mechanism is:
  - **Individualistic**: Each unit's assignment depends only on its own characteristics.
  - **Probabilistic**: Each unit has a non-zero chance of receiving either treatment or control.
  - **Unconfounded**: Given covariates, assignment does not depend on potential outcomes.
  - **Controlled by the Researcher**: The probability  $q$  is specified by the researcher.

# Bernoulli Trials - Probability of an Assignment Vector

For a Bernoulli trial, the probability of an assignment vector  $D$  for  $N$  units is given by:

$$\Pr(D|X, Y(0), Y(1)) = \prod_{i=1}^N \left( e(X_i)^{D_i} \cdot (1 - e(X_i))^{1-D_i} \right)$$

where:

- $D_i = 1$  if unit  $i$  is assigned to treatment,  $D_i = 0$  otherwise.
- $e(X_i)$ : Propensity Score for unit  $i$ .

If  $e(X_i) = q = 0.5$ , then  $\Pr(D|X, Y(0), Y(1)) = 0.5^N$ .

# Completely Randomized Experiment - Definition

A **completely randomized experiment** assigns a fixed number  $N_t$  of units to treatment, and the remaining  $N - N_t$  units to control.

- The assignment is achieved by randomly selecting  $N_t$  units from a pool of  $N$  units.
- Ensures a balanced distribution of treated and control units, with exactly  $N_t$  in treatment and  $N - N_t$  in control.
- Each unit's assignment is NOT independent of others, but the total number of treated units is fixed by design.
- The assignment mechanism is:
  - **Probabilistic**: Each unit has a positive probability of being selected for treatment or control.
  - **Unconfounded**: Given covariates, assignment does not depend on potential outcomes.
  - **Controlled by the Researcher**: The number  $N_t$  of treated units is specified by the researcher.

# Completely Randomized Experiment - Probability of an Assignment Vector

In a completely randomized experiment, the probability of an assignment vector  $D$  is:

$$\Pr(D|X, Y(0), Y(1)) = \begin{cases} \frac{1}{\binom{N}{N_t}} & \text{if } \sum_{i=1}^N D_i = N_t \\ 0 & \text{otherwise} \end{cases}$$

where  $N_t$  is the predetermined number of units assigned to treatment.

# Stratified Randomized Experiment - Definition

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- Performs complete randomization within each stratum, ensuring balanced treatment and control within each block.
- Reduces variability and improves the precision of causal inference estimates.
- The goal is to reduce variance in the estimator and increase the power of statistical tests, enhancing the study's ability to detect treatment effects.

# Stratified Randomized Experiment - Probability of an Assignment Vector

For a stratified randomized experiment with  $J$  blocks, the probability of an assignment vector  $D$  is:

$$\Pr(D|X, Y(0), Y(1)) = \prod_{j=1}^J \frac{1}{\binom{N(j)}{N_t(j)}}$$

where:

- $N(j)$ : Number of units in block  $j$ ,
- $N_t(j)$ : Number of treated units in block  $j$ .

# Paired Randomized Experiment - Definition

A **paired randomized experiment** is an extreme form of stratified randomization, where each block (or stratum) contains exactly two units.

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- Minimizes differences between treated and control units on covariates, reducing bias in the estimated treatment effect.
- Reduces variance in the estimator by closely aligning treatment and control units.
- Increases statistical power by enhancing the precision of the causal inference, making it easier to detect treatment effects.

# Paired Randomized Experiment - Probability of an Assignment Vector

For a paired randomized experiment with  $N/2$  pairs, the probability of an assignment vector  $D$  is:

$$\Pr(D|X, Y(0), Y(1)) = 2^{-\frac{N}{2}}$$

- Each unit within a pair has an equal probability of being assigned to treatment or control.



# Number of Possible Values for the Assignment Vector by Design and Sample Size

Type of Experiment and Design	Number of Possible Assignments	Number of Units (N) in Sample			
		4	8	16	32
Bernoulli trial	$2^N$	16	256	65,536	$4.2 \times 10^9$
Completely randomized experiment	$\binom{N}{N/2}$	6	70	12,870	$0.6 \times 10^9$
Stratified randomized experiment	$\left(\binom{N/2}{N/4}\right)^2$	4	36	4,900	$0.2 \times 10^9$
Paired randomized experiment	$2^{N/2}$	4	16	256	65,536

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# Stable Unit Treatment Value Assumption (SUTVA)

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- The potential outcomes for any unit do not vary with the treatments assigned to other units.

$$Y(1)_i = Y(1, 1)_i = Y(1, 0)_i \quad \text{for } i = 1, 2$$

**Example:** Under SUTVA, the potential outcomes for two units (1 and 2) would be consistent regardless of others' treatment status:

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- **Nonparametric Approach:** This method makes no assumptions about the distribution of the test statistic under the null hypothesis. (as t-test or ANOVA)
- **Flexibility** Both  $H_0$  and test statistic can be defined in various ways, making the method widely applicable.

Table 5.3: Cough Frequency for the First Six Units from the Honey Study

Unit	Potential Outcomes		Observed Variables		
	$Y_i(0)$	$Y_i(1)$	$D_i$	$X_i$	$Y_{\text{obs}}$
1		3	1	4	3
2		5	1	6	5
3		0	1	4	0
4	4		0	4	4
5	0		0	1	0
6	1		0	5	1

# Steps for the Exact Test

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- 2 Define the test statistic, calculating the difference in average outcomes by treatment status:

$$T_{\text{diff}} = \left| \left( \frac{\sum_{i:D_i=1} Y_{\text{obs},i}}{N_t} \right) - \left( \frac{\sum_{i:D_i=0} Y_{\text{obs},i}}{N_c} \right) \right|$$

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- 3 Compute the observed  $T_{\text{diff, obs}}$  from the data.

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- 1 Define  $H_0$ , e.g.,  $Y(1) = Y(0)$ .
- 2 Define the test statistic, calculating the difference in average outcomes by treatment status:

$$T_{\text{diff}} = \left| \left( \frac{\sum_{i:D_i=1} Y_{\text{obs},i}}{N_t} \right) - \left( \frac{\sum_{i:D_i=0} Y_{\text{obs},i}}{N_c} \right) \right|$$

- 3 Compute the observed  $T_{\text{diff, obs}}$  from the data.
- 4 Compute  $T_{\text{diff},k}$  for all possible assignment vectors  $D_k$ .



# Steps for the Exact Test

- 1 Define  $H_0$ , e.g.,  $Y(1) = Y(0)$ .
- 2 Define the test statistic, calculating the difference in average outcomes by treatment status:

$$T_{\text{diff}} = \left| \left( \frac{\sum_{i:D_i=1} Y_{\text{obs},i}}{N_t} \right) - \left( \frac{\sum_{i:D_i=0} Y_{\text{obs},i}}{N_c} \right) \right|$$

- 3 Compute the observed  $T_{\text{diff, obs}}$  from the data.
- 4 Compute  $T_{\text{diff},k}$  for all possible assignment vectors  $D_k$ .
- 5 Compute the p-value: approximate the p-value by the fraction of these  $K$  statistics that are as extreme as, or more extreme than, the observed  $T_{\text{diff, obs}}$ :

$$p = \frac{1}{K} \sum_{k=1}^K 1 \{ T_{\text{diff},k} \geq T_{\text{diff, obs}} \}$$

# Homework: Fisher Exact P-Value

- 1 Take the class and compute the Fisher Exact P-Value as before.
- 2 Modify the test statistic and compute the rank statistic instead. Compare results
- 3 Modify the null hypothesis and test the null hypothesis that the unit treatment effect is 10% Compare results
- 4 Compute Fisher Exact P-value using covariates (pp78 CIS)

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# Definition of Average Treatment Effect (ATE)

The **Average Treatment Effect (ATE)** is defined as:

$$\tau_{fs} = \frac{1}{N} \sum_{i=1}^N [Y_i(1) - Y_i(0)] = \bar{Y}(1) - \bar{Y}(0),$$

where:

$$\bar{Y}(1) = \frac{1}{N} \sum_{i=1}^N Y_i(1), \quad \bar{Y}(0) = \frac{1}{N} \sum_{i=1}^N Y_i(0).$$

## Definition of $\hat{\tau}_{\text{diff}}$

Define the estimator  $\hat{\tau}_{\text{diff}}$  as the difference of the sample means:

$$\hat{\tau}_{\text{diff}} = \bar{Y}_{\text{obs},t} - \bar{Y}_{\text{obs},c},$$

where:

$$\bar{Y}_{\text{obs},t} = \frac{1}{N_t} \sum_{i: W_i=1} Y_i^{\text{obs}}, \quad \bar{Y}_{\text{obs},c} = \frac{1}{N_c} \sum_{i: W_i=0} Y_i^{\text{obs}}.$$

# Theorem: Unbiasedness of $\hat{\tau}_{\text{diff}}$

**Theorem:** The estimator  $\hat{\tau}_{\text{diff}}$  is an unbiased estimator of the average treatment effect  $\tau_{fs}$ .

**Proof:**

# Definition of Sampling Variance of the Neyman Estimator

The sampling variance of  $\hat{\tau}_{\text{diff}}$  is:

$$V_W(\hat{\tau}_{\text{diff}}) = \frac{S_c^2}{N_c} + \frac{S_t^2}{N_t} - \frac{S_{tc}^2}{N},$$

where:

$$S_c^2 = \frac{1}{N-1} \sum_{i=1}^N (Y_i(0) - \bar{Y}(0))^2,$$

$$S_t^2 = \frac{1}{N-1} \sum_{i=1}^N (Y_i(1) - \bar{Y}(1))^2,$$

$$S_{tc}^2 = \frac{1}{N-1} \sum_{i=1}^N ([Y_i(1) - Y_i(0)] - \tau_{fs})^2.$$

## Theorem 6.2

**Theorem 6.2:** The sampling variance of  $\hat{\tau}_{\text{diff}}$  is given by:

$$V_W(\hat{\tau}_{\text{diff}}) = \frac{S_c^2}{N_c} + \frac{S_t^2}{N_t} - \frac{S_{tc}^2}{N}.$$

**Intuition:**



## Theorem 6.3

**Theorem 6.3:** If the treatment effect  $Y_i(1) - Y_i(0)$  is constant across units, then an unbiased estimator for the sampling variance is:

$$\hat{V}_{\text{Neyman}} = \frac{\hat{S}_c^2}{N_c} + \frac{\hat{S}_t^2}{N_t},$$

where  $s_c^2$  and  $s_t^2$  are sample variances calculated from the observed data.

**Estimation:**

# Confidence Intervals

To construct a  $(1 - \alpha) \times 100\%$  confidence interval for  $\tau_{fs}$ , we use:

$$CI_{1-\alpha}(\tau_{fs}) = \left[ \hat{\tau}_{\text{diff}} + z_{\alpha/2} \sqrt{\hat{V}}, \quad \hat{\tau}_{\text{diff}} + z_{1-\alpha/2} \sqrt{\hat{V}} \right],$$

where  $z_{\alpha/2}$  is the  $\alpha/2$  quantile of the standard normal distribution.

To test the null hypothesis  $H_0 : \tau_{fs} = 0$  against the alternative  $H_a : \tau_{fs} \neq 0$ , we compute the test statistic:

$$t = \frac{\hat{\tau}_{\text{diff}}}{\sqrt{\hat{V}}}.$$

We compare  $t$  to the critical values from the standard normal distribution.

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# Classical Additive Approach: Improving Precision Under Linearity

We start with the assumption that the conditional expectation function is exactly linear:

$$\mathbb{E}[Y \mid D, W] = D\alpha + \beta^\top X,$$

where:

- $D$  is the treatment indicator.
- $W$  represents pre-treatment covariates.
- $X = (1, W^\top)^\top$  includes an intercept and  $W$ .

# Centered Covariates and Covariate Balance

We assume that the covariates are centered:

$$\mathbb{E}[W] = 0.$$

By the assumption of covariate balance in randomized experiments:

$$\mathbb{E}[W \mid D = 1] = \mathbb{E}[W \mid D = 0].$$

# Average Treatment Effect under Linearity

Using centered covariates, we have:

$$\mathbb{E}[Y(0)] = \mathbb{E}[\mathbb{E}[Y \mid D = 0, X]] = \beta_1,$$

$$\mathbb{E}[Y(1)] = \mathbb{E}[\mathbb{E}[Y \mid D = 1, X]] = \beta_1 + \alpha.$$

Therefore, the Average Treatment Effect (ATE) is:

$$\delta = \mathbb{E}[Y(1)] - \mathbb{E}[Y(0)] = \alpha.$$

# Statistical Inference on the ATE

Even without assuming linearity, the projection coefficient  $\alpha$  recovers the ATE  $\delta$ .

Under regularity conditions, the Ordinary Least Squares (OLS) estimators satisfy:

$$\sqrt{n} \begin{pmatrix} \hat{\alpha} - \alpha \\ \hat{\beta}_1 - \beta_1 \end{pmatrix} \overset{approx}{\sim} \mathcal{N}(0, V),$$

where the covariance matrix  $V$  has components:

$$V_{11} = \frac{\mathbb{E}[\epsilon^2 \tilde{D}^2]}{(\mathbb{E}[\tilde{D}^2])^2},$$

$$V_{22} = \frac{\mathbb{E}[\epsilon^2 \tilde{I}^2]}{(\mathbb{E}[\tilde{I}^2])^2},$$

$$V_{12} = V_{21} = \frac{\mathbb{E}[\epsilon^2 \tilde{D} \tilde{I}]}{\mathbb{E}[\tilde{D}^2] \mathbb{E}[\tilde{I}^2]},$$

with:



# Relative Average Treatment Effect

We can also perform inference on the Relative ATE  $\alpha/\beta_1$ .  
Using the Delta Method, we have:

$$\sqrt{n} \left( \frac{\hat{\alpha}}{\hat{\beta}_1} - \frac{\alpha}{\beta_1} \right) \overset{approx}{\sim} \mathcal{N} \left( 0, G^\top V G \right),$$

where:

$$G = \begin{pmatrix} 1/\beta_1 \\ -\alpha/\beta_1^2 \end{pmatrix}.$$

# Improvement in Precision Under Linearity

When we do not include covariates, the OLS estimator  $\bar{\alpha}$  estimates  $\alpha$  in:

$$Y = \alpha D + \beta_1 + U, \quad \mathbb{E}[U] = \mathbb{E}[UD] = 0,$$

where:

$$U = \beta^\top (X - \mathbb{E}[X]) + \epsilon.$$

Under the linear model, including covariates improves precision:

$$V_{11} \leq \bar{V}_{11},$$

where  $\bar{V}_{11}$  is the variance when covariates are omitted.

# Limitations Without Linearity

Without the linearity assumption, the improvement in precision is not guaranteed.

- The variance  $V_{11}$  and  $\bar{V}_{11}$  are not generally comparable.
- Including covariates may increase the standard errors if the linear model is misspecified.

# The Interactive Approach: Capturing Heterogeneity

Consider the interactive linear model:

$$\mathbb{E}[Y \mid D, W] = \alpha^\top X D + \beta^\top X.$$

Here, the Conditional Average Treatment Effect (CATE) is:

$$\delta(W) = \mathbb{E}[Y(1) \mid W] - \mathbb{E}[Y(0) \mid W] = \alpha^\top X.$$

The Average Treatment Effect (ATE) is:

$$\delta = \mathbb{E}[\delta(W)] = \alpha_1,$$

where  $\alpha_1$  is the first component of  $\alpha$ .

# Estimation and Inference with Interactions

The coefficient  $\alpha$  is estimated from the linear projection:

$$Y = \alpha^\top (DX) + \beta^\top X + \epsilon, \quad \epsilon \perp (X, DX).$$

We can treat  $DX$  as a vector of technical treatments and apply standard inference methods.

# Advantages of the Interactive Approach

- Always delivers improvements in precision for estimating the ATE  $\delta$ , even if the linearity assumption does not hold.
- Allows for the discovery of treatment effect heterogeneity.

This result was demonstrated by Lin (2013).

# Conclusion

- Including pre-treatment covariates can improve the precision of ATE estimates in RCTs.
- The classical additive approach relies on linearity and may not always improve precision.
- The interactive approach, which includes interactions between treatment and covariates, always improves precision and uncovers heterogeneity.

# References I

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- Neyman, J. (1923). On the application of probability theory to agricultural experiments. essay on principles. section 9. *Statistical Science*, 5(4):465–472.