Design-Based Causal Inference with Missing Outcomes: Missingness Mechanisms, Imputation-Assisted Randomization Tests, and Covariate Adjustment

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Abstract

Design-based (also known as finite-population or randomization-based) causal inference is one of the most widely used frameworks for testing causal null hypotheses or inferring about causal parameters from experimental or observational data. The most significant merit of design-based causal inference is that its statistical validity only comes from the study design (e.g., randomization design in a randomized experiment) and does not require assuming any outcome-generating distributions or models. Although immune to model misspecification, design-based causal inference can still suffer from other data challenges, among which missingness in outcomes is a significant one. However, compared with model-based (also known as super-population or samplingbased) causal inference, outcome missingness in design-based causal inference is much less studied, largely due to the challenge that design-based causal inference does not assume any outcome distributions/models and, therefore, cannot directly adopt any existing model-based approaches for missing data. To fill this gap, we systematically study the missing outcomes problem in design-based causal inference. First, we use the potential outcomes framework to clarify the minimal assumption (concerning the outcome missingness mechanism) needed for conducting finite-population-exact randomization tests for the null effect (i.e., Fisher's sharp null) and that needed for constructing finite-population-exact confidence sets with missing outcomes. Second, we propose a general framework called "imputation and re-imputation" for conducting finite-population-exact randomization tests in design-based causal studies with missing outcomes. Our framework can incorporate any existing outcome imputation algorithms and meanwhile guarantee finite-population-exact type-I error rate control. Third, we extend our framework to conduct covariate adjustment in an exact randomization test with missing outcomes and to construct finite-population-exact confidence sets with missing outcomes. We conduct comprehensive simulation studies to examine exact type-I error rate control and gains in power using our framework. We have also developed an open-source Python package for implementation of our methods.

Keywords: Finite-population causal inference; Fisher's sharp null; Machine learning; Matching; Missing not at random; Randomization inference; Randomization test.

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1 Introduction

Design-based causal inference, also known as randomization-based or finite-population causal inference, has been commonly used in randomized experiments and observational studies and has become increasingly popular in the past two decades (Rosenbaum, 2002; Imbens and Rubin, 2015; Athey and Imbens, 2017; Li and Ding, 2017; Basse et al., 2019; Cohen and Fogarty, 2022; Zhang and Zhao, 2023). Compared with model-based (i.e., super-population or sampling-based) causal inference, design-based causal inference has two characteristics. First, the only probability distribution needed in design-based causal inference is the randomization of treatment assignments, which can be guaranteed by design, and no distributional assumptions on potential outcomes are needed. On the contrary, model-based causal inference typically needs to assume that the potential outcomes are identically and independently distributed realizations of some super-population distribution. Second, design-based causal inference focuses explicitly on the study subjects by conditioning on their potential outcomes and makes causal inference more relevant for the study subjects in hand. In contrast, model-based causal inference often focuses on some hypothetical super-population distribution. See Imbens and Rubin (2015), Athey and Imbens (2017), and Li and Small (2023) for detailed discussions on the advantages and limitations of design-based causal inference. Although design-based causal inference is immune to outcome model misspecification, it may still suffer from other data challenges, among which missingness in outcomes is a common one (Edgington and Onghena, 2007; Kennes et al., 2012; Rosenberger et al., 2019; Ivanova et al., 2022; Heussen et al., 2023). However, contrary to the rapidly growing literature on missing outcomes issues in model-based causal inference, existing literature on outcome missingness in design-based causal inference is scarce, largely due to the challenge that design-based causal inference does not impose any outcome models and, therefore, cannot directly incorporate existing model-based approaches to missing outcomes. Most existing approaches to design-based

causal inference with missing outcomes take one of the following three approaches: (i) using some non-informative missing outcome imputation (e.g., median, mean, or worst-case imputation) (Edgington and Onghena, 2007; Kennes et al., 2012; Rosenberger et al., 2019; Heussen et al., 2023; (ii) discarding the subjects with missing outcomes (including their covariate information) and reweighting the treatment assignments probabilities among the subjects with available outcome data based on the outcome missingness proportions among the treatment versus control groups (Edgington and Onghena, 2007; Kennes et al., 2012; Rosenberger et al., 2019; Heussen et al., 2023); and (iii) empirically embedding a modelbased multiple imputation approach into design-based inference (Ivanova et al., 2022). For these three approaches, the first and second approaches may substantially decrease statistical power as they ignore the fruitful covariate information in the datasets, and the third approach cannot guarantee exact type-I error rate control as its type-I error rate can be evidently above or below the pre-specified significance level even under some simple settings (Ivanova et al., 2022). To fill the aforementioned gap in the literature, we systematically investigate the missing outcomes issue in design-based causal inference. This work consists of the following three main parts:

- We use the potential outcomes framework to clarify the minimal assumption (concerning the outcome missingness mechanism) needed for conducting a finite-populationexact randomization test for Fisher's sharp null (one of the most fundamental and commonly considered null hypotheses in design-based causal inference) and that required for constructing a finite-population-exact confidence set for causal parameters of interest in deign-based causal inference with missing outcomes.
- Built on the above assumptions, we propose a general framework called "imputation and re-imputation" for constructing finite-population-exact randomization tests for testing Fisher's sharp null in the presence of missing outcomes. Our framework can incorporate any parametric or flexible machine learning missing outcomes imputation

algorithms to make full use of the covariate information to increase statistical power and meanwhile achieve finite-population-exact control of type-I error rate, even with unobserved covariates or interference in the outcome missingness mechanism.

• Built on the proposed framework, we show how to conduct covariate adjustment in an exact randomization test with missing outcomes to further increase power. We also discuss how to construct an exact confidence set by inverting the proposed imputation-assisted tests under additional assumptions.

The exact type-I error rate control and gains in power of our framework are examined via simulation studies. We also develop an open-source Python package I-ART (Imputation-Assisted Randomization Test) for the implementation of our methods.

2 Brief Review of Design-Based Causal Inference With Complete Outcome Data

We first review the classic notations and framework for design-based causal inference with complete outcome data (i.e., no missing outcomes) (Rosenbaum, 2002; Imbens and Rubin, 2015). Suppose there are $I \geq 1$ strata (or blocks, study centers, or matched sets), and $n_i \geq 2$ subjects in stratum *i*. In total, there are $N = \sum_{i=1}^{I} n_i$ subjects. For a given subject *j* in stratum *i*, let $Z_{ij} \in \{0, 1\}$ denote the treatment indicator ($Z_{ij} = 1$ if receiving treatment and $Z_{ij} = 0$ if receiving control), $\mathbf{x}_{ij} \in \mathbb{R}^{p_x}$ the observed covariates (i.e., observed pre-treatment variables), and $\mathbf{u}_{ij} \in \mathbb{R}^{p_y}$ the unobserved covariates. Suppose there are *K* outcomes of interest ($K \geq 1$), and let Y_{ijk} denote the *k*-th outcome of subject *j* in stratum *i* (we can omit *k* in the single outcome case). Let $\mathbf{Z} = (Z_{11}, \ldots, Z_{In_I}) \in \{0, 1\}^N$ denote the treatment indicator vector, $\mathbf{X} = \{\mathbf{x}_{ij} : i = 1, \ldots, I, j = 1, \ldots, n_i\}$ all the observed covariate information, $\mathbf{U} = \{\mathbf{u}_{ij} : i = 1, \ldots, I, j = 1, \ldots, n_i\}$ all the unobserved covariates, $\mathbf{Y}_k = (Y_{11k}, \ldots, Y_{In_Ik})$ all the *k*-th outcomes, and $\mathbf{Y} = (\mathbf{Y}_1, \ldots, \mathbf{Y}_K)$ all the *K* outcomes. Under the potential outcomes framework (Neyman, 1923; Rubin, 1974), we let $Y_{ijk}(1)$ and $Y_{ijk}(0)$ denote the potential k-th outcome of subject j in stratum i under treatment and that under control, respectively. Then, we have $Y_{ijk} = Z_{ij}Y_{ijk}(1) + (1 - Z_{ij})Y_{ijk}(0)$. In designbased causal inference, one of the most widely considered null hypotheses is Fisher's sharp null of no effect, of which the form (for the k-th outcome) is $H_{0,k}: Y_{ijk}(1) = Y_{ijk}(0)$ for all i, j. When there are multiple outcomes of interest (i.e., $K \ge 2$), the overall Fisher's sharp null is $H_0: \bigcap_{k=1}^K H_{0,k}$. The hypothesis testing part of our work focuses on Fisher's sharp null in the single and multiple outcome cases, which is often regarded as a first step in a causeand-effect analysis (Rosenbaum, 2002; Imbens and Rubin, 2015). For other widely used causal null hypotheses, such as Neyman's weak null hypothesis and no attributable effects, see Rosenbaum (2002) and Imbens and Rubin (2015) for detailed introductions. In designbased causal inference, all the potential outcomes $\mathcal{Y} = \{(Y_{ijk}(0), Y_{ijk}(1)) : i = 1, \dots, I, j = 1, \dots, I\}$ $1, \ldots, n_i, k = 1, \ldots, K$ are fixed values, and the only probability distribution that enters into statistical analysis is the randomization of treatment assignments (i.e., the probability distribution of **Z**) (Rosenbaum, 2002; Imbens and Rubin, 2015; Li and Ding, 2017). For example, in a stratified randomized experiment, researchers randomly assign the treatment to m_i subjects among the n_i total subjects in stratum *i*, where each m_i is a prespecified number. Specifically, let $\mathcal{Z} = \{ \mathbf{z} = (z_{11}, \dots, z_{In_I}) \in \{0, 1\}^N : \sum_{j=1}^{n_i} z_{ij} = m_i, i = 1, \dots, I \}$ denote all possible treatment assignments in a stratified randomized experiment, then we have

$$P(\mathbf{Z} = \mathbf{z} \mid \mathcal{Z}) = \prod_{i=1}^{I} {\binom{n_i}{m_i}}^{-1} \quad \text{for all } \mathbf{z} \in \mathcal{Z}.$$
 (1)

The study design (i.e., randomization design) (1) reduces to a completely randomized experiment when I = 1, and reduces to a paired randomized experiment when $n_i = 2$ and $m_i = 1$ for all *i*. In addition to randomized experiments, the randomization assumption (1) is also widely adopted in matched observational studies when assuming matching on observed covariates (i.e., $\mathbf{x}_{ij} = \mathbf{x}_{ij'}$ for all i, j, j') and no unobserved covariates (Rosenbaum, 2002). In addition to the study designs mentioned above, there are many other widely used study designs in design-based causal inference, such as Bernoulli randomized experiments (in which the probability of receiving the treatment for each study subject independently and identically follows Bernoulli(1/2)) and cluster randomized experiments (in which the unit of treatment assignments is cluster instead of individual); see Imbens and Rubin (2015) and Rosenbaum (2020) for detailed introductions to various study designs. After fixing a study design, we let Ω denote the collection of all possible treatment assignments and \mathcal{P} the probability distribution of \mathbf{Z} over Ω , induced by the study design (i.e., we have $\mathbf{Z} \mid \Omega \sim \mathcal{P}$), possibly conditional on \mathbf{X} (e.g., a randomized experiment stratified by some covariate such as age or gender). For example, in a stratified randomized experiment, the Ω is the aforementioned \mathcal{Z} , and the \mathcal{P} is the randomization distribution (1). In the single outcome case, given the observed value t of the test statistic $T(\mathbf{Z}, \mathbf{Y})$ (e.g., the Wilcoxon rank sum test, the permutational t-test, the Kolmogorov-Smirnov test, or their variants), we can calculate the finite-population-exact (permutational) p-value under Fisher's sharp null H_0 :

$$P(T(\mathbf{Z}, \mathbf{Y}) \ge t \mid \Omega, H_0) = \sum_{\mathbf{z} \in \Omega} \mathbb{1}\{T(\mathbf{Z} = \mathbf{z}, \mathbf{Y}) \ge t\} \times P(\mathbf{Z} = \mathbf{z} \mid \Omega), \text{ where } \mathbf{Z} \mid \Omega \sim \mathcal{P}.$$
(2)

When the sample size N is large, the exact p-value (2) can be approximated via the Monte Carlo method with arbitrary precision (Rosenbaum, 2002; Imbens and Rubin, 2015). In the multiple outcomes case, for testing overall Fisher's sharp null $H_0 : \bigcap_{k=1}^{K} H_{0,k}$, researchers can either directly use a test statistic $T(\mathbf{Z}, \mathbf{Y}_1, \dots, \mathbf{Y}_K)$ that combines all the K outcomes (e.g., some linear combination of K statistics calculated separately from the K individual outcomes (Rosenbaum, 2016)), or combine the K p-values based on (2) for each individual null $H_{0,k}$ using the Bonferroni correction or the Holm-Bonferroni method. In addition to testing the null effect, researchers may also want to construct confidence sets for causal parameters. For example, consider the following general class of treatment effect models:

$$Y_{ijk}(1) = f_k(Y_{ijk}(0), \vec{\beta}_k, \mathbf{x}_{ij}), \text{ for } i = 1, \dots, I, j = 1, \dots, n_i, k = 1, \dots, K,$$
(3)

where each f_k is a prespecified map from $Y_{ijk}(0)$ to $Y_{ijk}(1)$ that involve causal parameter(s) $\vec{\beta}_k$ and may also involve observed covariates \mathbf{x}_{ij} . For example, when $f_k = Y_{ijk}(0) + \beta_k$, the treatment effect model (3) is the additive constant effect model. When $f_k = \beta_k Y_{ijk}(0)$, the model (3) corresponds to the multiplicative effect model. When there are interaction terms between $\vec{\beta}_k$ and \mathbf{x}_{ij} , the model (3) allows for heterogeneous/individual treatment effects. Researchers can obtain a design-based (randomization-based) confidence set for the causal parameter(s) $(\vec{\beta}_1, \ldots, \vec{\beta}_K)$ via inverting the randomization tests for testing Fisher's sharp null with transformed outcomes $Y_{ijk,\vec{\beta}_k} = Z_{ij}Y_{ijk} + (1 - Z_{ij})f_k(Y_{ijk}, \vec{\beta}_k, \mathbf{x}_{ij})$ (because each $Y_{ijk,\vec{\beta}_k}$ will be invariant under any treatment assignments \mathbf{Z} if (3) holds); see Rosenbaum (2002, 2020) for detailed introduction.

3 Constructing Exact and Powerful Randomization Tests with Missing Outcomes

3.1 Clarifying the Outcome Missingness Mechanism for Design-Based Hypothesis Testing

In many practical studies, among the $K \ge 1$ outcomes, one or multiple of them have missingness. Let M_{ijk} denote the observed outcome missingness status (indicator) for the k-th outcome of subject ij: $M_{ijk} = 1$ if the k-th outcome of subject ij was missing, and $M_{ijk} = 0$ otherwise. That is, if we let Y_{ijk}^* denote the realized value (possibly with missingness) of the k-th outcome (henceforth called "realized k-th outcome") of subject ij, then we have $Y_{ijk}^* = Y_{ijk}$ if $M_{ijk} = 0$ (recall that each Y_{ijk} denotes the true, possibly unobserved, outcome) and $Y_{ijk}^* =$ "Missing" if $M_{ijk} = 1$. We let $\mathbf{M}_k = (M_{11k}, \ldots, M_{In_Ik})$, $\mathbf{M} = (\mathbf{M}_1, \ldots, \mathbf{M}_K)$, $\mathbf{Y}_k^* = (Y_{11k}^*, \dots, Y_{In_Ik}^*)$, and $\mathbf{Y}^* = (\mathbf{Y}_1^*, \dots, \mathbf{Y}_K^*)$. We still let $\mathbf{Y} = (\mathbf{Y}_1, \dots, \mathbf{Y}_K)$, where $\mathbf{Y}_k = (Y_{11k}, \dots, Y_{In_Ik})$, denote the true, possibly unobserved, outcomes. Since each missingness status M_{ijk} is a post-treatment variable, following the potential outcomes framework (Neyman, 1923; Rubin, 1974; Frangakis and Rubin, 1999), we let $M_{ijk}(\mathbf{z})$ denote the potential missingness status of the k-th outcome of subject ij under treatment assignments $\mathbf{z} \in \Omega$. Note that here we allow arbitrary interference in the missingness mechanism, i.e., we allow M_{ijk} to depend on $Z_{i'j'}$ for $i \neq i'$ and/or $j \neq j'$. Therefore, the observed missingness status $M_{ijk} = M_{ijk}(\mathbf{z})$ if $\mathbf{Z} = \mathbf{z}$. We then let $Y_{ijk}^*(\mathbf{z})$ denote the potential k-th realized outcome of subject ij, where $Y_{ijk}^*(\mathbf{z}) = Y_{ijk}(z_{ij})$ if $M_{ijk}(\mathbf{z}) = 0$ and $Y_{ijk}^*(\mathbf{z}) =$ "Missing" if $M_{ijk}(\mathbf{z}) = 1$. Therefore, the observed realized outcome $Y_{ijk}^* = Y_{ijk}^*(\mathbf{z})$ if $\mathbf{Z} = \mathbf{z}$. In design-based causal inference, the only source of randomness is the randomization of treatment assignments, and all the potential post-treatment variables, including the potential true outcomes $\mathcal{Y} =$ $\{(Y_{ijk}(1), Y_{ijk}(0)) : i = 1, \dots, I, j = 1, \dots, n_i, k = 1, \dots, K\}$, the potential outcome missingness indicators $\mathcal{M} = \{M_{ijk}(\mathbf{z}) : i = 1, \dots, I, j = 1, \dots, n_i, k = 1, \dots, K, \mathbf{z} \in \Omega\}$, and the potential realized outcomes $\mathcal{Y}^* = \{Y^*_{ijk}(\mathbf{z}) : i = 1, \dots, I, j = 1, \dots, n_i, k = 1, \dots, K, \mathbf{z} \in \Omega\},\$ are fixed values (Rosenbaum, 2002; Imbens and Rubin, 2015; Li and Ding, 2017; Li and Small, 2023).

There are many works clarifying the assumptions (concerning the outcome missingness mechanisms) required for model-based causal inference with missing outcomes. In contrast, parallel literature in design-based causal inference is scarce. To our knowledge, the only existing work that explicitly listed the assumptions concerning outcome missingness mechanism for randomization-based inference is Heussen et al. (2023). Our discussions on outcome missingness mechanisms in design-based causal inference differ from the related discussions in Heussen et al. (2023) in two aspects. First, our discussions on outcome missingness mechanisms are built on the potential outcomes framework (Neyman, 1923; Rubin, 1974; Frangakis and Rubin, 1999), which is the common language used in designbased causal inference. Second, our discussions explicitly clarify the minimal assumptions concerning the outcome missingness mechanism required for conducting finite-populationexact design-based hypothesis testing (see Assumption 1 stated below), and that required for constructing finite-population-exact design-based confidence sets with missing outcomes (see Assumption 2 in Section 5), respectively.

Assumption 1 (A General Outcome Missingness Mechanism for Design-Based Hypothesis Testing). Conditional on the finite-population dataset at hand, there exists an unknown map $\eta : \mathbb{R}^{N \times (p_x + p_y + K)} \to \{0, 1\}^{N \times K}$ such that $(\mathbf{M}_1, \dots, \mathbf{M}_K) = \eta(\mathbf{X}, \mathbf{U}, \mathbf{Y}_1, \dots, \mathbf{Y}_K).$

Assumption 1 claims that, conditional on the observed covariates \mathbf{X} and unobserved covariates \mathbf{U} (including unobserved pre-treatment error terms in the outcome missingness mechanism), the treatment assignments \mathbf{Z} can only affect the missingness status $(\mathbf{M}_1, \ldots, \mathbf{M}_K)$ through their effects on the post-treatment outcomes $(\mathbf{Y}_1, \ldots, \mathbf{Y}_K)$. We here make five important remarks on Assumption 1.

Remark 1. The missingness mechanism described in Assumption 1 belongs to the missingnot-at-random missingness mechanism (Little and Rubin, 2019) as it allows the missingness status to depend on unobserved covariates and missing outcomes among $(\mathbf{Y}_1, \ldots, \mathbf{Y}_K)$. For the same reason, Assumption 1 is untestable (without additional conditions) as it involves the missing outcomes.

Remark 2. Assumption 1 allows interference between study subjects – one subject's covariates and outcomes may affect another subject's outcome missingness status.

Remark 3. In the multiple outcomes case, Assumption 1 also allows interference between different outcomes of a given subject – one outcome can affect the missingness of another outcome. Such a scenario often occurs when the multiple outcomes are measured sequentially (Ivanova et al., 2022).

Remark 4. Assumption 1 allows each missingness status M_{ijk} to depend on Y_{ijk} , which is also called "self-censoring" in the missing outcome literature (d'Haultfoeuille, 2010; Wang

et al., 2014). Self-censoring in outcomes is common in practice, especially for self-reported outcomes.

Remark 5. The map η in Assumption 1 can be any unknown map, and there are no restrictions on its specific form.

In summary, the outcome missingness mechanism considered in Assumption 1 allows much flexibility as it allows dependence on observed and unobserved covariates, interference between study subjects and different outcomes, self-censoring, missing covariates, and arbitrary form of these dependencies (i.e., the map η can be an arbitrary unknown map). See Figures 1 and 2 for an illustration of the proposed outcome missingness mechanism in Assumption 1. Actually, as will be discussed in Remark 12 in Section 3.2, Assumption 1 is almost the weakest assumption that can guarantee finite-population-exact design-based hypothesis testing for Fisher's sharp null H_0 .



Figure 1: Single outcome case of Assumption 1.



Figure 2: Multiple outcomes case of Assumption 1.

3.2 Imputation-Assisted Randomization Tests for Design-Based Hypothesis Testing With Missing Outcomes: An Imputation and Re-Imputation Framework

As mentioned in Section 1, most previous studies on design-based hypothesis testing with missing outcomes choose between the following two classes of approaches:

• Class-One Approaches (in which covariate information is ignored): Imputing the missing outcomes with some non-informative values (e.g., median, mean, or the worst-case score) or simply removing the missing outcomes and then conducting a classic

randomization test with some reweighted treatment assignment probability based on the outcome missingness proportions among the treatment versus control groups (possibly with some simply adjusted randomization distribution) (Edgington and Onghena, 2007; Kennes et al., 2012; Rosenberger et al., 2019; Heussen et al., 2023).

• Class-Two Approaches (in which covariate information may be incorporated): Empirically embedding some model-based multiple imputation procedure into a design-based hypothesis testing procedure (Ivanova et al., 2022).

However, both of these two commonly considered classes of approaches have significant deficiencies. A deficiency of class-one approaches is that they ignored the fruitful covariate information and the potential association between the missingness status and outcome values, which can substantially decrease statistical power. A deficiency of class-two approaches is that they are some empirical strategies and cannot guarantee exact type-I error rate control. For example, as shown in Table 1 in Ivanova et al. (2022), such a strategy may either have conservative or inflated type-I error rate control even in some simple simulation settings.

To derive a design-based testing procedure with both finite-population-exact type-I error rate control and improved statistical power, we propose a general class of imputation-assisted randomization tests that can be implemented via an "imputation and re-imputation" framework described in Algorithm 1 below.

The imputation and re-imputation framework described in Algorithm 1 embeds a missing outcome imputation procedure into each permutation run of a randomization test. Compared with a randomization test incorporated with some empirical multiple imputation method (i.e., the aforementioned Approach 2), Algorithm 1 can achieve exact type-I error rate control, as will be shown in Theorem 1 and simulation studies in Section 3.3. Compared with a randomization test based on non-informative imputation such as median/mean imputation (i.e., the aforementioned Approach 1), Algorithm 1 can achieve **Algorithm 1:** An "imputation and re-imputation" framework for testing Fisher's sharp null.

- **Input:** The prespecified number of re-imputation runs L (e.g., L = 10,000). The observed treatment indicators \mathbf{Z} , the observed covariates \mathbf{X} , and the observed realized outcomes with missingness $\mathbf{Y}^* = (\mathbf{Y}_1^*, \dots, \mathbf{Y}_K^*)$ for the K outcomes $(K \ge 1)$. Note that \mathbf{Y}^* contains all the observed missingness status information $\mathbf{M} = (\mathbf{M}_1, \dots, \mathbf{M}_K)$.
 - 1. Use $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$ and some chosen algorithm \mathcal{G} (such as those based on regressions or flexible machine learning methods; see Remark 9) to obtain the full imputed outcomes $\widehat{\mathbf{Y}}$:

$$\mathcal{G}: (\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*) \mapsto \widehat{\mathbf{Y}} = (\widehat{\mathbf{Y}}_1, \dots, \widehat{\mathbf{Y}}_K).$$
 (The Imputation Step)

We then calculate the $t = T(\mathbf{Z}, \hat{\mathbf{Y}})$, where T is some chosen test statistic (T can be any test statistics based on \mathbf{Z} and $\hat{\mathbf{Y}}$; see Remark 11).

- 2. For each $l = 1, \ldots, L$, do the following steps:
 - (a) Randomly generate $\mathbf{Z}^{(l)}$ according to the randomization design \mathcal{P} (see Remark 10).
 - (b) Obtain the full imputed outcomes $\widehat{\mathbf{Y}}^{(l)}$ based on $(\mathbf{Z}^{(l)}, \mathbf{X}, \mathbf{Y}^*)$ and algorithm \mathcal{G} :

$$\mathcal{G}: (\mathbf{Z}^{(l)}, \mathbf{X}, \mathbf{Y}^*) \mapsto \widehat{\mathbf{Y}}^{(l)} = (\widehat{\mathbf{Y}}_1^{(l)}, \dots, \widehat{\mathbf{Y}}_K^{(l)}).$$
 (The Re-Imputation Step)

- (c) Calculate the $T^{(l)} = T(\mathbf{Z}, \widehat{\mathbf{Y}}^{(l)})$ for the *l*-th re-imputation run and store the value.
- 3. Approximate the finite-population-exact p-value under Fisher's sharp null H_0 via:

$$\widehat{p} = \frac{1}{L} \sum_{l=1}^{L} \mathbb{1}\{T^{(l)} \ge t\}.$$

Output: The approximate finite-population-exact *p*-value \hat{p} under Fisher's sharp null H_0 .

much higher statistical power by making full use of the observed covariates and outcomes in each imputation and re-imputation step, as will also be shown in Section 3.3. Here are some additional remarks.

Remark 6. For the full imputed outcomes $\widehat{\mathbf{Y}} = (\widehat{\mathbf{Y}}_1, \dots, \widehat{\mathbf{Y}}_K)$, in which $\widehat{\mathbf{Y}}_k = (\widehat{Y}_{11k}, \dots, \widehat{Y}_{In_Ik})$, we have each $\widehat{Y}_{ijk} = Y_{ijk}^* = Y_{ijk}$ if missing indicator $M_{ijk} = 0$ and \widehat{Y}_{ijk} equals the outcome value imputed by some chosen imputation algorithm \mathcal{G} if $M_{ijk} = 1$.

Remark 7. When the sample size is tiny (i.e., $|\Omega|$ is small) and the outcome imputation algorithm \mathcal{G} is deterministic (see Remark 9 for more details), instead of randomly drawing a treatment assignment $\mathbf{Z}^{(l)}$ from the randomization distribution \mathcal{P} in each simulation run, we can directly list all possible treatment assignments Ω and calculate the finite-populationexact *p*-value based on the imputation and re-imputation procedure; see also formula (4) in Lemma 1.

Remark 8. We here use a simple example in the single outcome case to illustrate the necessity of the re-imputation step in Algorithm 1. Consider a completely randomized experiment with four subjects (indexed with ID 1–4), among which there are two treated subjects and two control subjects. Therefore, there are six possible treatment assignments: $\mathbf{Z}^{(1)} = (1, 0, 1, 0), \, \mathbf{Z}^{(2)} = (1, 0, 0, 1), \, \mathbf{Z}^{(3)} = (0, 1, 1, 0), \, \mathbf{Z}^{(4)} = (0, 1, 0, 1), \, \mathbf{Z}^{(5)} = (1, 1, 0, 0),$ and $\mathbf{Z}^{(6)} = (0, 0, 1, 1).$ Let $\Omega = \{\mathbf{Z}^{(1)}, \mathbf{Z}^{(2)}, \mathbf{Z}^{(3)}, \mathbf{Z}^{(4)}, \mathbf{Z}^{(5)}, \mathbf{Z}^{(6)}\}$. Suppose the observed treatment assignment $\mathbf{Z} = \mathbf{Z}^{(1)} = (1, 0, 1, 0)$ and the corresponding observed realized outcomes $\mathbf{Y}^* = (1, 0, \text{NA}, \text{NA}),$ where "NA" means "Missing." We consider two imputation-assisted randomization tests: one based on the imputation and re-imputation approach and one based on a one-shot imputation procedure (i.e., without re-imputation). For both approaches, we consider Fisher's exact test statistic (i.e., the number of treated subjects with observed or imputed outcome being 1).

We first calculate the exact *p*-value under sharp null H_0 using the imputation and re-imputation approach. We use a natural outcome imputation algorithm \mathcal{G} defined as the following: for each treated (or control) subject whose outcome is missing, we impute its missing outcome using the mean value of all the non-missing outcomes among the treated (or control) subjects; if all the treated (or control) subjects' outcomes are missing, we impute 0.5 for all the missing outcomes of the treated (or control) subjects. This imputation algorithm is perhaps the most natural one when there is no covariate information. For each treatment assignment $\mathbf{Z}^{(n)}(n = 1, \dots, 6)$, based on the observed realized outcomes $\mathbf{Y}^* = (1, 0, \text{NA}, \text{NA})$ (fixed under sharp null H_0 and Assumption 1 for different treatment assignments) and imputation algorithm \mathcal{G} , the corresponding imputed outcomes $\widehat{\mathbf{Y}}^{(n)}$ can be obtained and the corresponding imputation-assisted Fisher's exact test statistic $T^{(n)} =$ $\mathbf{Z}^{(n)}(\widehat{\mathbf{Y}}^{(n)})^T$ can be calculated, which are summarized in Figure 3. We take Datasets 1, 3, and 5 as illustrative examples for showing the detailed imputation process. For Dataset 1 in Figure 3 (the observed dataset), based on the imputation algorithm \mathcal{G} defined above and the training dataset $(\mathbf{Z}^{(1)}, \mathbf{Y}^*)$, the imputed outcome for a treated subject will be 1 (because the only treated subject with available outcome data have outcome 1) and that for a control subject will be 0 (because the only control subject with available outcome data have outcome 0). Instead, for Dataset 3 in Figure 3 (a permuted dataset), based on the imputation algorithm \mathcal{G} and the training dataset $(\mathbf{Z}^{(3)}, \mathbf{Y}^*)$, the imputed outcome for a treated subject will be 0 (because the only treated subject with available outcome data have outcome 0 in the permuted dataset $(\mathbf{Z}^{(3)}, \mathbf{Y}^*)$ and that for a control subject will be 1 (because the only control subject with available outcome data have outcome 1 in the permuted dataset). For Dataset 5 in Figure 3 (a permuted dataset), based on the imputation algorithm \mathcal{G} and the training dataset $(\mathbf{Z}^{(5)}, \mathbf{Y}^*)$, the imputed outcome for a control subject will be 0.5 because there is no control subject with available outcome data in the permuted dataset $(\mathbf{Z}^{(5)}, \mathbf{Y}^*)$, so we simply assign the imputed outcome 0.5 (the mean/median value of 0 and 1) to control subjects with missing outcomes. Meanwhile, for Dataset 5, both treated subjects have available outcome data, and the "imputed" outcomes for them are naturally set as the values of their available outcomes. See Figure 3 for

Dataset 1 (Observed)						Dataset 2 (Permuted)				Dataset 3 (Permuted)						
ID	$\mathbf{Z}^{(1)}$	\mathbf{Y}^*	$\widehat{\mathbf{Y}}^{(1)}$	$\widehat{\mathbf{Y}}_{*}^{(1)}$		ID	$\mathbf{Z}^{(2)}$	\mathbf{Y}^*	$\widehat{\mathbf{Y}}^{(2)}$	$\widehat{\mathbf{Y}}_{*}^{(2)}$		ID	$\mathbf{Z}^{(3)}$	\mathbf{Y}^*	$\widehat{\mathbf{Y}}^{(3)}$	$\widehat{\mathbf{Y}}_{*}^{(3)}$
1	1	1	1	1		1	1	1	1	1		1	0	1	1	1
2	0	0	0	0		2	0	0	0	0		2	1	0	0	0
3	1	NA	1	1		3	0	NA	0	0		3	1	NA	0	1
4	0	NA	0	0		4	1	NA	1	1		4	0	NA	1	0
$T^{(1)} = 2; T^{(1)}_* = 2$					$T^{(2)} = 2; T^{(2)}_* = 2$						$T^{(3)} = 0; T^{(3)}_* = 1$					
Dataset 4 (Permuted)					Dataset 5 (Permuted)						Dataset 6 (Permuted)					
ID	$\mathbf{Z}^{(4)}$	\mathbf{Y}^*	$\widehat{\mathbf{Y}}^{(4)}$	$\widehat{\mathbf{Y}}_{*}^{(4)}$		ID	$\mathbf{Z}^{(5)}$	\mathbf{Y}^*	$\widehat{\mathbf{Y}}^{(5)}$	$\widehat{\mathbf{Y}}_{*}^{(5)}$		ID	$\mathbf{Z}^{(6)}$	\mathbf{Y}^*	$\widehat{\mathbf{Y}}^{(6)}$	$\widehat{\mathbf{Y}}_{*}^{(6)}$
1	0	1	1	1		1	1	1	1	1		1	0	1	1	1
2	1	0	0	0		2	1	0	0	0		2	0	0	0	0
3	0	NA	1	0		3	0	NA	0.5	0		3	1	NA	0.5	1
4	1	NA	0	1		4	0	NA	0.5	0		4	1	NA	0.5	1
$T^{(4)} = 0; T^{(4)}_* = 1$					$T^{(5)} = 1; T_*^{(5)} = 1$					$T^{(6)} = 1; T_*^{(6)} = 2$						

Figure 3: An illustrative example of the necessity of re-imputation.

the imputed results for all six datasets. Therefore, the one-sided (greater than) exact p-value reported by the imputation and re-imputation approach given the observed dataset (Dataset 1 in Figure 3) is

$$P(T \ge T^{(1)} \mid \Omega, H_0) = |\{\mathbf{Z}^{(n)} : T^{(n)} = \mathbf{Z}^{(n)} (\widehat{\mathbf{Y}}^{(n)})^T \ge 2, n = 1, \dots, 6\}| / |\Omega| = 1/3.$$

We then calculate the exact *p*-value under sharp null H_0 using a one-shot imputation procedure (i.e., without re-imputation). Specifically, to facilitate a fair comparison, we still use the aforementioned algorithm \mathcal{G} and use a training dataset (e.g., that obtained from sample splitting or some external dataset) that is the same as that used in the imputation step of the imputation and re-imputation approach (i.e., the observed Dataset 1 in Figure 3). This will give us an "oracle" imputation model \mathcal{G}_* : we impute value 1 for missing outcomes among the treated subjects and value 0 for missing outcomes among the control subjects. Therefore, for Dataset 1 (the observed dataset), the imputed outcomes given by the oneshot imputation approach are the same as those given by the imputation and re-imputation approach, i.e., we have $\mathcal{G}_*(\mathbf{Z}^{(1)}, \mathbf{Y}^*) = \mathcal{G}(\mathbf{Z}^{(1)}, \mathbf{Y}^*) = (1, 0, 1, 0)$. However, an intrinsic difference between the imputation and re-imputation approach using algorithm \mathcal{G} and a one-shot imputation approach using model \mathcal{G}_* is that the former one will re-train the imputation model using \mathcal{G} for each of the six permutations of \mathbf{Z} (i.e., the re-imputation step). In contrast, the latter one will not re-train the imputation model and will stick to \mathcal{G}_* (i.e., imputing the missing outcome as value 1 for treated subjects with missing outcomes and value 0 for control subjects with missing outcomes) in each permutation. We can then calculate the imputed outcomes reported by this one-shot imputation approach (denoted as $\widehat{\mathbf{Y}}_*^{(n)}$) based on each permutated dataset $(\mathbf{Z}^{(n)}, \mathbf{Y}^*)$, as well as the corresponding Fisher's exact test statistic $T_*^{(n)} = \mathbf{Z}^{(n)}(\widehat{\mathbf{Y}}_*^{(n)})^T$; see Figure 3 for details. Therefore, the one-sided (greater than) exact *p*-value reported by the one-shot imputation approach is

$$P(T_* \ge T_*^{(1)} \mid \Omega, H_0) = |\{\mathbf{Z}^{(n)} : T_*^{(n)} = \mathbf{Z}^{(n)} (\widehat{\mathbf{Y}}_*^{(n)})^T \ge 2, n = 1, \dots, 6\}| / |\Omega| = 1/2,$$

which is much larger than the p-value 1/3 reported by the imputation and re-imputation approach.

Note that the above example is intended to illustrate the necessity of re-imputation instead of to show the superior performance of the imputation and re-imputation approach over classic randomization tests based on non-informative imputation such as median or mean imputation. If we consider using median/mean imputation in the above example, the imputed outcomes will always be (1, 0, 0.5, 0.5) for the six possible treatment assignments $\mathbf{Z}^{(n)}(n = 1, ..., 6)$, and the corresponding *p*-value is 1/3, which is the same as the *p*value reported by the imputation and re-imputation approach. This is because there is no covariate information in this simple example, which is the critical source of improved power using the imputation and re-imputation approach compared with non-informative imputation. In Section 3.3, we will conduct comprehensive simulation studies to show that, compared with the commonly used non-informative imputation approach in design-based causal inference, the imputation and re-imputation approach can substantially increase statistical power by making better use of the covariate information.

Remark 9. The algorithm \mathcal{G} for outcome imputation can be chosen from any existing imputation algorithms, such as k-nearest neighbors imputation (Cover and Hart, 1967), hot-deck imputation (Andridge and Little, 2010), or chained equations imputation based on linear regressions (e.g., Bayesian ridge regression) or flexible machine learning methods (e.g., random forest, boosting, or deep neural networks) (Pedregosa et al., 2011; Little and Rubin, 2019; Kim and Shao, 2021). Moreover, the output $\hat{\mathbf{Y}}$ of the algorithm \mathcal{G} can be either deterministic (e.g., K-nearest neighbor imputation or non-random versions of hot-deck imputation or chained equations imputation, in which $\hat{\mathbf{Y}} = \mathcal{G}(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$ is a deterministic function of $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$) or stochastic (e.g., random versions of hot-deck imputation or chained equations imputation, in which $\hat{\mathbf{Y}} = \mathcal{G}(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$ is a random variable conditional on $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$).

Remark 10. The randomization design \mathcal{P} can be any prespecified probability distribution over Ω , such as complete randomization, stratified randomization, paired randomization, Bernoulli randomization, cluster randomization, and so on.

Remark 11. The test statistic $T = T(\mathbf{Z}, \widehat{\mathbf{Y}})$ can be any test statistic based on \mathbf{Z} and $\widehat{\mathbf{Y}}$. For example, in the single outcome case (i.e., K = 1), if we choose the permutational t-test statistic, then $T(\mathbf{Z}, \widehat{\mathbf{Y}}) = \sum_{i=1}^{I} \sum_{j=1}^{n_i} Z_{ij} \widehat{Y}_{ij}$. If we choose the Wilcoxon rank sum test statistic, then $T(\mathbf{Z}, \widehat{\mathbf{Y}}) = \sum_{i=1}^{I} \sum_{j=1}^{n_i} Z_{ij} \operatorname{rank}(\widehat{Y}_{ij})$ where $\operatorname{rank}(\widehat{Y}_{ij}) = \sum_{i'=1}^{I} \sum_{j'=1}^{n_{i'}} \mathbb{1}\{\widehat{Y}_{ij} \ge \widehat{Y}_{i'j'}\}$. In the multiple outcomes case, we can choose $T(\mathbf{Z}, \widehat{\mathbf{Y}})$ to be some linear combination of the K test statistics T_1, \ldots, T_K , among which each T_k focuses on the k-th outcome. For example, we can let $T_k(\mathbf{Z}, \widehat{\mathbf{Y}}_k) = \sum_{i=1}^{I} \sum_{j=1}^{n_i} Z_{ij} \operatorname{rank}(\widehat{Y}_{ijk})$ where $\operatorname{rank}(\widehat{Y}_{ijk})$ where $\operatorname{rank}(\widehat{Y}_{ijk}) = \sum_{i'=1}^{I} \sum_{j'=1}^{n_{i'}} \mathbb{1}\{\widehat{Y}_{ijk} \ge \widehat{Y}_{i'j'k}\}$, and then let $T = \sum_{k=1}^{K} \lambda_k T_k$, where $\lambda_1, \ldots, \lambda_K$ are prespecified weights (Rosenbaum, 2016). In addition to this combined statistic strategy, an alternative way to hypothesis testing in the multiple outcomes case is to combine the K p-values

 $\hat{p}_1, \ldots, \hat{p}_K$ through the Bonferroni correction or the Holm-Bonferroni method, where each \hat{p}_k is reported by applying the individual test T_k in Algorithm 1.

The following Theorem 1 shows that, under Assumption 1, as the number of reimputation runs L increases, the p-value \hat{p} reported by Algorithm 1 converges almost surely to the finite-population-exact p-value for Fisher's sharp null, and the convergence rate is $O_p(1/\sqrt{L})$.

Theorem 1. Consider the imputation and re-imputation framework in Algorithm 1 paired with any chosen outcome imputation algorithm $\mathcal{G} : (\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*) \mapsto \widehat{\mathbf{Y}} = (\widehat{\mathbf{Y}}_1, \dots, \widehat{\mathbf{Y}}_K)$ and any chosen test statistic $T = T(\mathbf{Z}, \widehat{\mathbf{Y}})$. Let t be the observed value of T based on the observed data $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$ and outcome imputation algorithm \mathcal{G} . Let $p = P(T \ge t \mid \Omega, H_0)$ denote the true finite-population-exact p-value under Fisher's sharp null H_0 . For the approximate p-value \widehat{p} reported by Algorithm 1, under Assumption 1, we have

$$\widehat{p} \xrightarrow{a.s.} p$$
 as the number of re-imputation runs $L \to \infty$.

Moreover, for any $\epsilon > 0$ and for all L, we have

$$P(|\hat{p} - p| \ge \epsilon) \le 2\exp(-2L\epsilon^2).$$

The rationale of Theorem 1 is straightforward: under the outcome missingness mechanism in Assumption 1 and Fisher's sharp null H_0 , the realized outcomes \mathbf{Y}^* (including the information of missingness status \mathbf{M}) are invariant under different treatment assignments \mathbf{Z} . Therefore, we can drive an explicit formula of the finite-population-exact *p*-value in terms of the observed data and the output of outcome imputation algorithm \mathcal{G} , as shown in Lemma 1 below.

Lemma 1. Let $\mathcal{G} : (\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*) \mapsto \widehat{\mathbf{Y}}$ be any outcome imputation algorithm for obtaining the imputed outcomes $\widehat{\mathbf{Y}}$, and $T(\mathbf{Z}, \widehat{\mathbf{Y}})$ be any test statistic based on \mathbf{Z} and $\widehat{\mathbf{Y}}$. Let $P(T \ge$ $t \mid \Omega, H_0$) be the finite-population-exact p-value under Fisher's sharp null H_0 given the observed value t of T. If the missing outcome imputation algorithm \mathcal{G} is deterministic (i.e., $\widehat{\mathbf{Y}} = \mathcal{G}(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$ is a deterministic value given $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$), we have

$$P(T \ge t \mid \Omega, H_0) = \sum_{\mathbf{z} \in \Omega} \left[\mathbb{1}\{T(\mathbf{Z} = \mathbf{z}, \mathcal{G}(\mathbf{Z} = \mathbf{z}, \mathbf{X}, \mathbf{Y}^*)) \ge t\} \times P(\mathbf{Z} = \mathbf{z} \mid \Omega) \right], \quad (4)$$

If the missing outcome imputation algorithm \mathcal{G} is stochastic (i.e., $\widehat{\mathbf{Y}} = \mathcal{G}(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$ is a random variable conditional on $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$), we have

$$P(T \ge t \mid \Omega, H_0) = \sum_{\mathbf{z} \in \Omega} \left[P\{T(\mathbf{Z} = \mathbf{z}, \mathcal{G}(\mathbf{Z} = \mathbf{z}, \mathbf{X}, \mathbf{Y}^*)) \ge t\} \times P(\mathbf{Z} = \mathbf{z} \mid \Omega) \right].$$
(5)

Based on the derived formula in Lemma 1, we can then use Monte Carlo simulations equipped with a missing data imputation algorithm \mathcal{G} (i.e., the re-imputation step) to approximate the *p*-value $P(T \ge t \mid \Omega, H_0)$ as in Algorithm 1, which gives us the result in Theorem 1. The detailed proofs of all theorems and lemmas in this paper can be found in Appendix A in the online supplementary materials. Finally, we give one additional essential remark.

Remark 12. As shown in Lemma 1 and the above arguments, the key point of guaranteeing finite-population-exact type-I error rate control for testing Fisher's sharp null H_0 with missing outcomes is that, under H_0 , the observed realized outcomes \mathbf{Y}^* (including the observed missingness status \mathbf{M}) are invariant under different treatment assignments \mathbf{Z} . This also agrees with the principle of testing H_0 with complete outcome data in the classic randomization test literature (Rosenbaum, 2002; Imbens and Rubin, 2015). Therefore, Assumption 1 is almost the weakest assumption required for ensuring finite-populationexact type-I error rate control for testing H_0 because if the missingness indicators \mathbf{M} also depend on some post-treatment variables other than $(\mathbf{X}, \mathbf{U}, \mathbf{Y})$, then \mathbf{M} can vary with different treatment assignments \mathbf{Z} even under H_0 , which makes it infeasible to construct a finite-population-exact randomization test for H_0 without additional strong assumptions.

3.3 Simulation Studies

In this section, we conduct simulation studies to investigate the type-I error rate control and power of imputation-assisted randomization tests constructed by the imputation and reimputation framework described in Algorithm 1 and compare them with (i) the commonly used non-informative imputation approach (e.g., median imputation) for randomization test with missing outcomes (which is, to our knowledge, the only existing class of designbased hypothesis testing approaches that can ensure finite-population-exact type-I error rate control without discarding the study subjects with missing outcomes in the testing procedure) and (ii) the oracle randomization test based on complete outcome data. Because our framework is design-based (i.e., finite-population-based) and does not require assuming any specific outcome-generating distributions or models, the data-generating processes described below are only intended for automatically generating finite-population datasets for our simulation studies.

We consider a stratified randomized experiment with I strata. In each stratum, there are 10 subjects, among which we randomly assign 5 subjects to receive the treatment and 5 subjects to receive the control. We generate a five-dimensional observed covariates vector $(x_{ij1}, \ldots, x_{ij5})$ for each subject j in stratum i using the following data generating process:

$$(x_{ij1}, x_{ij2}) \stackrel{\text{i.i.d.}}{\sim} \mathcal{N}\left(\begin{pmatrix} \frac{1}{2} \\ -\frac{1}{3} \end{pmatrix}, \begin{pmatrix} 1 & \frac{1}{2} \\ \frac{1}{2} & 1 \end{pmatrix} \right), (x_{ij3}, x_{ij4}) \stackrel{\text{i.i.d.}}{\sim} \text{Laplace}\left(\begin{pmatrix} 0 \\ \frac{1}{\sqrt{3}} \end{pmatrix}, \begin{pmatrix} 1 & \frac{1}{\sqrt{2}} \\ \frac{1}{\sqrt{2}} & 1 \end{pmatrix} \right),$$

and $x_{ij5} \stackrel{\text{i.i.d.}}{\sim}$ Bernoulli(1/3). We also generate an aggregate unobserved covariate u_{ij} (including the unobserved error term in the outcome missingness generating process) for each subject ij according to $u_{ij} \stackrel{\text{i.i.d.}}{\sim} N(0, 0.5)$. In the outcome-generating process, we include a stratum-level random effect α_i and an individual-level random effect ϵ_{ij} , where

 $\alpha_i \stackrel{\text{i.i.d.}}{\sim} N(0, 0.1)$ and $\epsilon_{ij} \stackrel{\text{i.i.d.}}{\sim} N(0, 0.2)$. Therefore, the total variance that cannot be captured by the observed data is $\operatorname{var}(u_{ij}) + \operatorname{var}(\alpha_i) + \operatorname{var}(\epsilon_{ij}) = 0.8$. We consider the following four data-generating models consisting of a model for generating the true outcome Y_{ij} and a model for generating the missingness status M_{ij} , with increasing complexity:

• Model 1 (Constant treatment effect; linear model for the true outcome; linear selection model for the missingness status; without interference in the missingness mechanism):

$$\begin{split} Y_{ij} &= \beta Z_{ij} + \frac{1}{\sqrt{5}} \sum_{p=1}^5 x_{ijp} + u_{ij} + \alpha_i + \epsilon_{ij}, \\ M_{ij} &= \mathbbm{1} \left\{ \frac{1}{\sqrt{5}} \sum_{p=1}^5 p x_{ijp} + Y_{ij} + u_{ij} > \lambda \right\}. \end{split}$$

• Model 2 (Constant treatment effect; non-linear model for the true outcome; non-linear selection model for the missingness status; without interference in the missingness mechanism):

$$Y_{ij} = \beta Z_{ij} + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} x_{ijp} + \frac{1}{5} \sum_{p=1}^{5} \sum_{p'=1}^{5} x_{ijp} \sigma (1 - x_{ijp'}) + u_{ij} + \alpha_i + \epsilon_{ij},$$
$$M_{ij} = \mathbb{1} \left\{ \frac{1}{\sqrt{5}} \sum_{p=1}^{5} p x_{ijp} + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} p \cos(x_{ijp}) + 10\sigma(Y_{ij}) + u_{ij} > \lambda \right\}.$$

• Model 3 (Heterogeneous treatment effects; non-linear model for the true outcome; non-linear selection model for the missingness status; without interference in the missingness mechanism):

$$Y_{ij} = \beta Z_{ij} \left(1 + x_{ij1} + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} |x_{ijp}| \right) + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} x_{ijp} + \frac{1}{5} \sum_{p=1}^{5} \sum_{p'=1}^{5} x_{ijp} \sigma (1 - x_{ijp'}) + u_{ij} + \alpha_i + \epsilon_{ij}$$
$$M_{ij} = \mathbb{1} \left\{ \frac{1}{\sqrt{5}} \sum_{p=1}^{5} p x_{ijp} + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} p \cos(x_{ijp}) + 10\sigma(Y_{ij}) + u_{ij} > \lambda \right\}.$$

• Model 4 (Heterogeneous treatment effects; non-linear model for the true outcome;

non-linear selection model for the missingness status; with interference in the missingness mechanism):

$$Y_{ij} = \beta Z_{ij} \left(1 + x_{ij1} + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} |x_{ijp}| \right) + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} x_{ijp} + \frac{1}{5} \sum_{p=1}^{5} \sum_{p'=1}^{5} x_{ijp} \sigma (1 - x_{ijp'}) + u_{ij} + \alpha_i + \epsilon_{ij},$$
$$M_{ij} = \mathbb{1} \left\{ \frac{1}{\sqrt{5}} \sum_{p=1}^{5} p x_{ijp} + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} p \cos(x_{ijp}) + 10\sigma(Y_{ij}) + u_{ij} + \frac{1}{10} \sum_{j=1}^{10} x_{ij1} + \frac{1}{10} \sum_{j=1}^{10} Y_{ij} > \lambda \right\}.$$

In Models 1–4, each λ is a tuning parameter for controlling the outcome missingness rate (we set λ such that the missingness rate is 50% for each simulated dataset), and the σ function is defined as $\sigma(x) = \exp(x)/(1 + \exp(x))$. For each model, we consider two scenarios: a small sample size scenario in which we set the total sample size N = 50 (corresponding to setting I = 5) and a large sample size scenario in which we set N = 1000 (corresponding to setting I = 100). In each model and each simulation scenario, we implement the following four methods for design-based hypothesis testing with missing outcomes:

- Method 1 (Median Imputation): Classic randomization test based on median imputation.
- Method 2 (Algo 1 Linear): Imputation-assisted randomization test based on the imputation and re-imputation framework described in Algorithm 1, in which we set the embedded outcome imputation algorithm *G* to be chained equations imputation based on linear regression (more specifically, Bayesian ridge regression) (Pedregosa et al., 2011; Little and Rubin, 2019).
- Method 3 (Algo 1 Boosting): Imputation-assisted randomization test based on the imputation and re-imputation framework described in Algorithm 1, in which we set the embedded outcome imputation algorithm \mathcal{G} to be chained equations imputation based on some boosting algorithm in machine learning. Specifically, in the small sample size scenario (when N = 50), the boosting algorithm we choose is the well-known

XGBoost algorithm (Chen and Guestrin, 2016), which is a gradient boosting decision tree algorithm that has relatively robust performance even under a small or moderate sample size. In the large sample size scenario (when N = 1000), the boosting algorithm we choose is the widely used LightGBM algorithm (Ke et al., 2017), which is a newer gradient boosting decision tree algorithm that has less computational cost than XGBoost and is particularly suitable for implementing more re-imputation runs (i.e., larger L) in Algorithm 1 with large datasets.

• Method 4 (Oracle): Classic randomization test with the complete outcome data (oracle/true outcomes).

For all the above four methods, we use an adjusted Wilcoxon rank sum test statistic $T_{adj}(\mathbf{Z}, \widehat{\mathbf{Y}})$ and one-sided testing procedure, in which $T_{adj}(\mathbf{Z}, \widehat{\mathbf{Y}}) = \sum_{i=1}^{I} \sum_{j=1}^{n_i} Z_{ij} \operatorname{rank}_{adj}(\widehat{Y}_{ij})$, where $\operatorname{rank}_{adj}(\widehat{Y}_{ij}) = \sum_{i'j':M_{i'j'}=0} \mathbb{1}\{Y_{ij} \ge Y_{i'j'}\}$ if $M_{ij} = 0$ and $\operatorname{rank}_{adj}(\widehat{Y}_{ij}) = \sum_{i'j':M_{i'j'}=1} \mathbb{1}\{Y_{ij} \ge Y_{i'j'}\}$ if $M_{ij} = 1$. That is, the $T_{adj}(\mathbf{Z}, \widehat{\mathbf{Y}})$ considers the ranks among the non-missing outcomes and those among the imputed values for the missing outcomes separately. Therefore, the statistical scores (i.e., adjusted ranks) contributed by the subjects with non-missing outcome data are unchanged under Methods 1–4, and the differences in the statistical power of Methods 1–4 can only be due to their different performances in imputing missing outcomes. This facilitates a more fair and transparent comparison study.



Figure 4: Simulated Type-I error rate (when effect size $\beta = 0$) and power (when effect size $\beta > 0$) of Methods 1–4 under Models 1–4 with sample size N = 50 and N = 1000, based on level $\alpha = 0.05$.

The simulated type-I error rate (obtained via setting the effect size $\beta = 0$ in the outcomegenerating model, with level $\alpha = 0.05$ and 10,000 simulated datasets) and the simulated power (corresponding to $\beta > 0$ in the outcome generation, with level $\alpha = 0.05$ and 2,000 simulated datasets) are reported in Figure 4. The simulation results reported in Figure 4 deliver the following messages. First, the imputation and re-imputation framework can achieve finite-population-exact type-I error rate control (corresponding to the $\beta = 0$ case) with either linear imputation models (Method 2) or flexible machine learning imputation algorithm (Method 3) under all the Models 1–4 (ranging from linear to non-linear outcome models and missingness models, with or without interference in the outcome missingness mechanism, in the presence of unobserved covariates). This confirms the theoretical guarantee of finite-population-exact type-I error rate control of the imputation and re-imputation framework, as stated in Theorem 1. Second, the imputation and re-imputation framework can substantially increase statistical power by using an imputation algorithm (either based on a simple linear model or a flexible machine learning model) to incorporate the covariates information into a randomization test with imputed outcomes. Third, when the sample size is small (e.g., N = 50), the imputation and re-imputation approach based on a linear model (i.e., Method 1) is more powerful (sometimes only slightly more powerful) than that based on a nonparametric boosting algorithm under Models 1–4. Fourth, when the sample size is large (e.g., N = 1000), as the outcome model and missingness model become more and more complex from Model 1 to Models 3 and 4 (i.e., from linear to nonlinear models, from constant treatment effects to heterogeneous treatment effects), incorporating a flexible nonparametric machine learning algorithm such as boosting into the imputation and re-imputation approach would be more and more promising to have higher statistical power than incorporating a simple linear imputation model into the imputation and re-imputation approach, and the gains in power tend to become larger as the model complexity (i.e., degree of nonlinearity and/or effect heterogeneity) increases.

We then consider the multiple missing outcomes case. Specifically, we consider the following data-generating process for the three true outcomes Y_{ij1} , Y_{ij2} , and Y_{ij3} and the corresponding missingness indicators M_{ij1} , M_{ij2} , and M_{ij3} :

• Model 5 (Multiple outcomes; Heterogeneous treatment effects; non-linear model for the true outcomes; non-linear selection model for the missingness status; with interference in the missingness mechanism):

$$\begin{split} Y_{ij1} &= \beta Z_{ij} \left(\frac{1}{4} + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} \sigma(x_{ijp}) \right) + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} x_{ijp} + \frac{1}{5} \sum_{p=1}^{5} \sum_{p'=1}^{5} x_{ijp} x_{ijp'} + \sin(u_{ij}) + \alpha_{i1} + \epsilon_{ij1} + Y_{ij2} \\ Y_{ij2} &= \beta Z_{ij} \left(1 + x_{ij1} + u_{ij} \right) - \frac{1}{5} \sum_{p=1}^{5} \sum_{p'=1}^{5} x_{ijp} \sigma(1 - x_{ijp'}) + \alpha_{i2} + \epsilon_{ij2}, \\ Y_{ij3} &= \beta Z_{ij} \frac{1}{\sqrt{5}} \sum_{p=1}^{5} |x_{ijp}| + \frac{1}{\sqrt{5}} \sum_{p=1}^{5} x_{ijp} + \frac{1}{5\sqrt{5}} \sum_{p=1}^{5} \sum_{p'=1}^{5} \sum_{p'=1}^{5} x_{ijp} x_{ijp'} \sigma(x_{ijp''}) + u_{ij} + \alpha_{i3} + \epsilon_{ij3}, \\ M_{ij1} &= \mathbbm{1} \left\{ \frac{1}{\sqrt{5}} \sum_{p=1}^{5} \sigma(x_{ijp}) + \frac{1}{5} \sum_{p=1}^{5} \sum_{p'=1}^{5} x_{ijp} x_{ijp'} + 5\sigma(Y_{ij1}) + u_{ij} > \lambda_1 \right\}, \\ M_{ij2} &= \mathbbm{1} \left\{ \frac{1}{\sqrt{5}} \sum_{p=1}^{5} x_{ijp}^3 + \frac{1}{5} \sum_{p=1}^{5} \sum_{p'=1}^{5} x_{ijp} x_{ijp'} + \frac{5}{2} \sum_{k=1}^{3} \sigma(Y_{ijk}) + \sigma(1 - u_{ij}) > \lambda_2 \right\}, \\ M_{ij3} &= \mathbbm{1} \left\{ \frac{1}{\sqrt{5}} \sum_{p=1}^{5} px_{ijp} + \frac{1}{5\sqrt{5}} \sum_{p=1}^{5} \sum_{p'=1}^{5} \sum_{p'=1}^{5} x_{ijp} x_{ijp'} x_{ijp''} + \frac{5}{3} \sum_{k=1}^{3} \sigma(Y_{ijk}) + \sin(u_{ij}^2) > \lambda_3 \right\}. \end{split}$$

In Model 5, the stratum-level random effect α_{ik} are generated through: $\alpha_{ik} \stackrel{\text{i.i.d.}}{\sim} N(0, 0.1)$ for each stratum *i* and for each k = 1, 2, 3, and the individual-level random effect ϵ_{ijk} are generated through the following process: $\epsilon_{ijk} \stackrel{\text{i.i.d.}}{\sim} N(0, 0.2)$, for each subject *ij* and each outcome k = 1, 2, 3. Therefore, the total variance of the three terms that cannot be captured by observed data is $\operatorname{var}(u_{ij}) + \operatorname{var}(\alpha_{ik}) + \operatorname{var}(\epsilon_{ijk}) = 0.8$. We set $(\lambda_1, \lambda_2, \lambda_3)$ such that the missingness rate is 50% for each outcome and for each simulated dataset. In Table 1, we report the simulated power of Methods 1–4 (using the Bonferroni correction to adjust for multiple outcomes) under Model 5, setting level $\alpha = 0.05$. Table 1 shows, similar to the single outcome case in Models 1–4, the imputation and re-imputation approach (Methods 2 and 3) can still substantially increase the statistical power of a randomization test with multiple missing outcomes.

		N = 50		N = 1000				
	$\beta = 0.36$	$\beta=0.48$	$\beta = 0.6$	$\beta = 0.06$	$\beta = 0.09$	$\beta = 0.12$		
Method 1 (Median Imputation)	0.244	0.351	0.470	0.208	0.358	0.564		
Method 2 (Algo 1 – Linear)	0.348	0.508	0.670	0.274	0.516	0.755		
Method 3 (Algo 1 – Boosting)	0.333	0.459	0.621	0.333	0.648	0.861		
Method 4 (Oracle)	0.713	0.876	0.959	0.596	0.909	0.994		

Table 1: Simulated power of Methods 1–4 under Model 5 (the multiple outcomes case), with total sample size N = 50 and N = 1000. Each number is based on level $\alpha = 0.05$ and 2.000 simulated datasets.

4 Covariate Adjustment in Randomization Tests with Missing Out-

comes

In design-based causal inference with complete outcome data, researchers often seek to further improve statistical power via incorporating a covariate adjustment procedure in a randomization test (Rosenbaum, 2002; Small et al., 2008; Lin, 2013; Fogarty et al., 2021; Zhao and Ding, 2021). For example, for testing Fisher's sharp null of no effect with complete outcome data, a common strategy for covariate adjustment is to conduct a randomization test based on residuals obtained from some working outcome prediction model (e.g., some regression model or flexible machine learning model) based only on the observed covariates, without looking at the treatment assignments information (Rosenbaum, 2002; Small et al., 2008). In this section, we show how to conduct covariate adjustment under the proposed imputation and re-imputation framework, of which the detailed procedure is summarized in Algorithm 2 below.

We here give two remarks on Algorithm 2.

Remark 13. The working model \mathcal{H} for obtaining the predicted outcomes can be either deterministic (e.g., ordinary linear regression or k-nearest neighbors algorithm) or stochastic (e.g., Bayesian regression or random forest). Let $\widetilde{Y}_{ijk} = \widetilde{Y}_{ijk}(\mathbf{X}, \mathbf{Y}^*)$ denote the predicted value of the k-th outcome of subject ij based on $(\mathbf{X}, \mathbf{Y}^*)$, then $\widetilde{\mathbf{Y}}_k = (\widetilde{Y}_{11k}, \ldots, \widetilde{Y}_{In_Ik})$ and $\widetilde{\mathbf{Y}} = (\widetilde{\mathbf{Y}}_1, \ldots, \widetilde{\mathbf{Y}}_K)$. Algorithm 2: An "imputation and re-imputation" framework with covariate adjustment.

- **Input:** The prespecified number of re-imputation runs L (e.g., L = 10,000). The observed treatment indicators \mathbf{Z} , the observed covariates \mathbf{X} , and the observed realized outcomes with missingness $\mathbf{Y}^* = (\mathbf{Y}_1^*, \dots, \mathbf{Y}_K^*)$ for the K outcomes $(K \ge 1)$. Note that \mathbf{Y}^* contains all the observed missingness status information $\mathbf{M} = (\mathbf{M}_1, \dots, \mathbf{M}_K)$.
 - 1. (a) Use $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$ and some chosen imputation algorithm \mathcal{G} (such as those based on regressions or machine learning methods; recall Remark 9) to obtain the full imputed outcomes $\widehat{\mathbf{Y}}$:

$$\mathcal{G}: (\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*) \mapsto \widehat{\mathbf{Y}} = (\widehat{\mathbf{Y}}_1, \dots, \widehat{\mathbf{Y}}_K).$$
 (The Imputation Step)

(b) Use $(\mathbf{X}, \widehat{\mathbf{Y}})$ and some chosen outcome prediction model \mathcal{H} (e.g., \mathcal{H} can be any regression model or machine learning model; see Remark 13) to get the predicted outcomes $\widetilde{\mathbf{Y}}$ (without using the treatment information \mathbf{Z}):

 $\mathcal{H}: (\mathbf{X}, \widehat{\mathbf{Y}}) \mapsto \widetilde{\mathbf{Y}} = (\widetilde{\mathbf{Y}}_1, \dots, \widetilde{\mathbf{Y}}_K), \quad \text{(Covariate Adjustment after Imputation)}$ where each $\widetilde{\mathbf{Y}}_k = (\widetilde{Y}_{11k}, \dots, \widetilde{Y}_{In_Ik})$ denote the predicted k-th outcomes based on \mathcal{H} and $(\mathbf{X}, \widehat{\mathbf{Y}}).$

- (c) Calculate the $t = T(\mathbf{Z}, \vec{\epsilon})$, where $\vec{\epsilon} = \widehat{\mathbf{Y}} \widetilde{\mathbf{Y}}$ is the residuals vector and T is some chosen test statistic based on $(\mathbf{Z}, \vec{\epsilon})$ (T can be any test statistics).
- 2. For each $l = 1, \ldots, L$, do the following steps:
 - (a) Randomly generate $\mathbf{Z}^{(l)}$ according to the randomization design \mathcal{P} .
 - (b) Obtain the full imputed outcomes $\widehat{\mathbf{Y}}^{(l)}$ based on $(\mathbf{Z}^{(l)}, \mathbf{X}, \mathbf{Y}^*)$ and algorithm \mathcal{G} :

$$\mathcal{G}: (\mathbf{Z}^{(l)}, \mathbf{X}, \mathbf{Y}^*) \mapsto \widehat{\mathbf{Y}}^{(l)} = (\widehat{\mathbf{Y}}_1^{(l)}, \dots, \widehat{\mathbf{Y}}_K^{(l)}).$$
 (The Re-Imputation Step)

(c) Use $(\mathbf{X}, \widehat{\mathbf{Y}}^{(l)})$ and working outcome prediction model \mathcal{H} to get the predicted outcomes $\widetilde{\mathbf{Y}}$ (without using the treatment information \mathbf{Z}):

 $\mathcal{H}: (\mathbf{X}, \widehat{\mathbf{Y}}^{(l)}) \mapsto \widetilde{\mathbf{Y}}^{(l)} = (\widetilde{\mathbf{Y}}_{1}^{(l)}, \dots, \widetilde{\mathbf{Y}}_{K}^{(l)}), \quad \text{(Covariate Adjustment after Re-Imputation)}$ where each $\widetilde{\mathbf{Y}}_{k}^{(l)} = (\widetilde{Y}_{11k}^{(l)}, \dots, \widetilde{Y}_{In_{I}k}^{(l)})$ denote the predicted k-th outcomes based

- on \mathcal{H} and $(\mathbf{X}, \widehat{\mathbf{Y}}^{(l)})$. (d) Calculate the $T^{(l)} = T(\mathbf{Z}, \vec{\epsilon}^{(l)})$ and store the value, where $\vec{\epsilon}^{(l)} = \widehat{\mathbf{Y}}^{(l)} - \widetilde{\mathbf{Y}}^{(l)}$ is
- 3. Approximate the finite-population-exact *p*-value under Fisher's sharp null H_0 via: $\widehat{p}_{adj} = \frac{1}{L} \sum_{l=1}^{L} \mathbb{1}\{T^{(l)} \ge t\}.$

Output: The approximate finite-population-exact *p*-value \hat{p}_{adj} under Fisher's sharp null H_0 .

the residuals vector for the *l*-th permutation.

Remark 14. Let $\hat{\epsilon}_{ijk} = \hat{Y}_{ijk} - \tilde{Y}_{ijk}$ be the residuals obtained via conducting covariate adjustment with imputed outcomes, i.e., the difference in the imputed outcome \hat{Y}_{ijk} using the full observed data $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$ and the predicted outcome \tilde{Y}_{ijk} only using $(\mathbf{X}, \mathbf{Y}^*)$, of the k-th outcome of subject ij. Let $\vec{\epsilon}_k = (\hat{\epsilon}_{11k}, \ldots, \hat{\epsilon}_{In_Ik})$ and $\vec{\epsilon} = (\vec{\epsilon}_1, \ldots, \vec{\epsilon}_K)$.

The following Theorem 2 shows that, under Assumption 1, as the number of reimputation runs L increases, the *p*-value \hat{p}_{adj} reported by Algorithm 2 converges to the true finite-population-exact *p*-value for Fisher's sharp null with rate $O_p(1/\sqrt{L})$.

Theorem 2. Let \mathcal{G} be any outcome imputation algorithm used in Algorithm 2 and \mathcal{H} any prediction model for covariate adjustment based on imputed outcomes used in Algorithm 2. Let $T = T(\mathbf{Z}, \vec{\epsilon})$ be any test statistic based on \mathbf{Z} and residuals $\vec{\epsilon}$ after covariate adjustment, and let t denote the observed value of T based on the observed data $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}^*)$. Let $p_{adj} =$ $P(T \geq t \mid \Omega, H_0)$ denote the finite-population-exact p-value (with covariate adjustment) under Fisher's sharp null H_0 . For the approximate p-value \hat{p}_{adj} reported by the imputation and re-imputation approach with covariate adjustment described in Algorithm 2, under Assumption 1, we have

$$\widehat{p}_{adj} \xrightarrow{a.s.} p_{adj}$$
 as the number of re-imputation runs $L \to \infty$.

Moreover, for any $\epsilon > 0$ and for all L, we have

$$P(|\widehat{p}_{adj} - p_{adj}| \ge \epsilon) \le 2\exp(-2L\epsilon^2).$$

In Appendix B in the online supplementary materials, we conduct a simulation study to investigate the type-I error rate and power of the imputation and re-imputation approach with covariate adjustment described in Algorithm 2. The simulation results show that the imputation and re-imputation approach with covariate adjustment can (i) achieve exact type-I error rate control for any imputation algorithm \mathcal{G} and covariate adjustment model \mathcal{H} and (ii) may further improve the statistical power of the imputation and re-imputation approach in many settings. See Appendix B for detailed results and discussions.

5 Finite-Population-Exact Confidence Sets With Missing Outcomes Via Inverting An Imputation-Assisted Randomization Test

In addition to testing the null effect, researchers are often concerned about constructing confidence sets for causal parameters of interest. As reviewed in Section 1, in design-based causal inference with complete outcome data, these two tasks (i.e., hypothesis testing and construction of a confidence set) are equivalent: a finite-population-exact confidence set can be obtained via inverting exact randomization tests (Rosenbaum, 2002). However, such equivalence no longer holds in the presence of missing outcomes. As we will show in this section, constructing an exact confidence set requires a stronger assumption than those needed for exact hypothesis testing (i.e., Assumption 1). We state such an assumption below.

Assumption 2 (A General Outcome Missingness Mechanism for Design-Based Confidence Sets). Conditional on the finite-population dataset at hand, there exists an unknown map $\phi : \mathbb{R}^{N \times (p_x + p_y)} \to \{0, 1\}^{N \times K}$ such that $(\mathbf{M}_1, \dots, \mathbf{M}_K) = \phi(\mathbf{X}, \mathbf{U}).$

Assumption 2 can imply an outcome missingness mechanism considered in Heussen et al. (2023) (specifically, the Condition 1 in Heussen et al. (2023), which states that outcome missingness is stochastically independent of treatment assignments) under the potential outcomes framework. It claims that missingness status can be fully captured by the observed and unobserved covariates (including unobserved pre-treatment error terms in the outcome missingness mechanism), which can be tested based on the observed data. Assumption 2 is a much stronger assumption than Assumption 1 as the missingness status no longer depends on the post-treatment outcomes and, therefore, will not be affected by treatment assignments, which may not hold in some settings. However, as we will show in Remark 15, Assumption 2 is already nearly the minimal assumption required for constructing an exact confidence set in design-based causal inference with missing outcomes.

The following Theorem 3 shows that, under Assumption 2, we can construct a finitepopulation-exact confidence set in design-based causal inference with missing outcomes (the proof is in Appendix A.3).

Theorem 3. Consider the same setting as that in Theorem 1 and further assume that Assumption 2 holds. Consider the following null hypothesis with prespecified functions f_1, \ldots, f_K and prespecified values of the causal parameters $\vec{\beta}_0 = (\vec{\beta}_{01}, \ldots, \vec{\beta}_{0K})$:

$$H_{\vec{\beta}_0}: Y_{ijk}(1) = f_k(Y_{ijk}(0), \vec{\beta}_{0k}, \mathbf{x}_{ij}), \text{ for } i = 1, \dots, I, j = 1, \dots, n_i, k = 1, \dots, K.$$
(6)

Let $\mathbf{Y}_{\vec{\beta}_0}^* = (\mathbf{Y}_{1,\vec{\beta}_01}^*,\ldots,\mathbf{Y}_{K,\vec{\beta}_{0K}}^*)$ be the transformed realized outcomes (with missingness), in which we have $\mathbf{Y}_{k,\vec{\beta}_{0k}}^* = (Y_{11k,\vec{\beta}_{0k}}^*,\ldots,Y_{In_Ik,\vec{\beta}_{0k}}^*)$ where $Y_{ijk,\vec{\beta}_{0k}}^* = \text{``Missing'' if } M_{ijk} = 1$ and $Y_{ijk,\vec{\beta}_{0k}}^* = Z_{ij}Y_{ijk}^* + (1 - Z_{ij})f_k(Y_{ijk}^*,\vec{\beta}_{0k},\mathbf{x}_{ij}) = Z_{ij}Y_{ijk} + (1 - Z_{ij})f_k(Y_{ijk},\vec{\beta}_{0k},\mathbf{x}_{ij})$ if $M_{ijk} = 0$. Let $\hat{p}(\vec{\beta}_0)$ denote the approximate p-value reported by Algorithm 1 (without covariate adjustment after imputation) or Algorithm 2 (with covariate adjustment after imputation) based on the inputs $(\mathbf{Z}, \mathbf{X}, \mathbf{Y}_{\vec{\beta}_0}^*)$. For the prespecified level $\alpha \in (0, 1)$, we define the set $CI = \{\vec{\beta}_0 : \hat{p}(\vec{\beta}_0) \ge 1 - \alpha\}$. As the number of re-imputation runs L increases, for the unknown true causal parameters $\vec{\beta} = (\vec{\beta}_1, \ldots, \vec{\beta}_K)$ that satisfy $Y_{ijk}(1) = f_k(Y_{ijk}(0), \vec{\beta}_k, \mathbf{x}_{ij})$ for all i, j, k, we have

$$\inf_{L \to \infty} P(\vec{\beta} \in CI \mid \Omega) \ge 1 - \alpha.$$

We here briefly explain the rationale of Theorem 3. Note that, under Assumption 2, the missingness indicators \mathbf{M} are invariant under any treatment assignments \mathbf{Z} and any $H_{\vec{\beta}_0}$. Then, under $H_{\vec{\beta}_0}$, the transformed realized outcomes $\mathbf{Y}^*_{\vec{\beta}_0}$ are invariant under different \mathbf{Z} . In other words, testing $H_{\vec{\beta}_0}$ is equivalent to testing Fisher's sharp null of no effect with the transformed outcomes $\mathbf{Y}^*_{\vec{\beta}_0}$. Therefore, applying Algorithm 1 or Algorithm 2 to $\mathbf{Y}^*_{\vec{\beta}_0}$ can produce (approximate) finite-population-exact *p*-values $\hat{p}(\vec{\beta}_0)$ for testing each $H_{\vec{\beta}_0}$, and an

approximate finite-population-exact confidence set CI can be obtained via inverting the *p*-values $\hat{p}(\vec{\beta}_0)$.

Remark 15. As discussed above, a key component of the proof of Theorem 3 is that the missingness indicators \mathbf{M} are invariant under any $\vec{\beta}_0$ in $H_{\vec{\beta}_0}$, which holds under Assumption 2, but may not hold under Assumption 1. Specifically, under Assumption 1 (i.e., missingness indicators are allowed to depend on \mathbf{Y}), the observed missingness indicators under the observed treatment assignments \mathbf{Z} is $(\mathbf{M}_1(\mathbf{Z}), \ldots, \mathbf{M}_K(\mathbf{Z})) = \eta(\mathbf{X}, \mathbf{U}, \mathbf{Y}(\mathbf{Z}))$ and those under another set of treatment assignments \mathbf{Z}' will be $(\mathbf{M}_1(\mathbf{Z}'), \ldots, \mathbf{M}_K(\mathbf{Z}')) =$ $\eta(\mathbf{X}, \mathbf{U}, \mathbf{Y}(\mathbf{Z}'))$. Note that under $H_{\vec{\beta}_0}$, the $\mathbf{Y}(\mathbf{Z}')$ may differ from $\mathbf{Y}(\mathbf{Z})$ when $\mathbf{Z}' \neq \mathbf{Z}$. Therefore, without additional assumptions (i.e., Assumption 2), missingness status \mathbf{M} may not be invariant under different treatment assignments \mathbf{Z} , and a finite-population-exact randomization test for $H_{\vec{\beta}_0}$ is infeasible.

6 Discussion

In this paper, we systematically investigated design-based causal inference with missing outcomes, including (i) clarifying the assumption concerning the outcome missingness mechanism required for conducting finite-population-exact hypothesis testing for Fisher's sharp null of no effect and that required for constructing finite-population-exact confidence sets, (ii) proposing a universal imputation and re-imputation framework for conducting finitepopulation-exact hypothesis testing for Fisher's sharp null, which allow researchers to incorporate any flexible outcome imputation algorithms to increase statistical power while ensuring finite-population-exact type-I error rate control, and (iii) extending the imputation and re-imputation framework to allow covariate adjustment with missing outcomes and to construct finite-population-exact confidence sets with missing outcomes.

Our results also suggest future research directions. For example, for the hypothesis testing part, our work focuses on Fisher's sharp null, which is one of the most widely considered null hypotheses in design-based causal inference and is often considered as a first step in a cause-and-effect analysis (Imbens and Rubin, 2015). However, our work has not explicitly discussed other common null hypotheses in design-based causal inference, such as Neyman's weak null (Neyman, 1923; Imbens and Rubin, 2015; Zhao and Ding, 2021; Cohen and Fogarty, 2022) and attributable effects (Rosenbaum, 2001, 2002; Hansen and Bowers, 2009). When the outcome variable is discrete, previous studies (Rigdon and Hudgens, 2015; Li and Ding, 2016; Rosenbaum, 2001; Hansen and Bowers, 2009) showed that testing Neyman's weak null or attributable effects is equivalent to testing a sequence of carefully formulated Fisher's sharp null hypotheses. This may shed light on using our framework to help study other null hypotheses (e.g., Neyman's weak null with discrete outcomes and attributable effects) in design-based causal inference with missing outcomes.

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Supplementary Material

Online supplementary material includes the technical proofs, additional details and simulation studies, and related discussions.

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