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Model-free causal inference of binary experimental data

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Abstract

For binary experimental data, we discuss randomization-based inferential procedures that do not need to invoke any modeling assumptions. In addition to the classical method of moments, we also introduce model-free likelihood and Bayesian methods based solely on the physical randomization without any hypothetical super population assumptions about the potential outcomes. These estimators have some properties superior to moment-based ones such as only giving estimates in regions of feasible support. Due to the lack of identification of the causal model, we also propose a sensitivity analysis approach that allows for the characterization of the impact of the association between the potential outcomes on statistical inference.

KEYWORDS

attributable effect, average causal effect, Bayesian inference, completely randomized experiment, likelihood, sensitivity analysis

1 | INTRODUCTION

In randomized experiments, the outcome of interest is often binary, in which case the resulting data can be summarized by a 2×2 table. In this paper, we give an in-depth discussion of estimating causal effects for those 2×2 tables generated by completely randomized experiments. Under the potential outcomes framework (Neyman, 1923; Rubin, 1974), each unit has pretreatment potential outcomes corresponding to the potential treatments that unit could receive. Finite population causal inference (e.g., Imbens & Rubin, 2015; Li & Ding, 2017; Rosenbaum, 2002) focuses on the experimental units at hand and treats all potential outcomes as fixed with the randomization of treatment assignment as the only source of randomness. This view allows for weak modeling assumptions and inferential methods that are valid due to the randomization mechanism itself rather than any stated belief in a data generating process. Furthermore,

by focusing on the finite population, the precision of the usual difference-in-means estimator is greater than that of comparable infinite population models. Unfortunately, the uncertainty of the estimator depends on the association between the potential outcomes, an unidentifiable quantity that can complicate finite population inference (Imbens & Rubin, 2015; Neyman, 1923).

Binary outcomes, however, lend enough structure to the problem that these issues can be somewhat circumvented. Because of the discrete nature of the problem, there are only a small number of possible types of units that could exist, which allows for two things. First, we can achieve sharper bounds on the variance of the moment estimator. Second, we can actually implement model-free likelihood and Bayesian procedures for treatment effects. These estimators have superior performance to the usual moment estimators because they exploit the structure of the problem in order to limit possible estimates to a restricted parameter space. In particular, the observed data assign zero likelihood outside a well-defined region of possibilities, and so, procedures based on this likelihood will not return any of these impossible estimates. Moment estimators, on the other hand, could return such values.

It is well known that the association between the potential outcomes plays an important role in estimating the average causal effect. Different approaches have been used to address this difficulty. Some restrict attention to testing the sharp null hypothesis of zero causal effect for all experimental units (Copas, 1973; Fisher, 1935). Some enumerate all possible combinations of the potential outcomes in order to construct exact confidence intervals (Li & Ding, 2016; Rigdon & Hudgens, 2015b). Some derive bounds on the variances of the estimators over all possible randomizations using the marginal distributions (Aronow, Green, & Lee, 2014; Ding & Dasgupta, 2016; Fogarty, Mikkelsen, Gaieski, & Small, 2016; Robins, 1988). Some assume nonnegative individual causal effects, allowing causal effects to be estimated directly (Rosenbaum, 2001) or use structures such as constant shifts (Rosenbaum, 2002) or dilations to dictate all the individual outcomes (Rosenbaum, 1999). Recent work on Bayesian inference imputes missing potential outcomes based on their posterior predictive distributions, which requires modeling the potential outcomes as binomial samples from a hypothetical infinite population (Ding & Dasgupta, 2016).

The methods we present in this paper are distinct from these. Extending Copas (1973), we show that the randomization itself allows for obtaining a likelihood function and, consequently, a Bayesian posterior distribution (under a prior distribution) without any outcome modeling assumptions. To acknowledge the weak identifiability of the association between potential outcomes, we advocate a sensitivity analysis strategy to show the dependences of the repeated-sampling, likelihood, and Bayesian inferences on a sensitivity parameter. All proofs have been relegated to the Appendix and supplementary material.

2 | POTENTIAL OUTCOMES, CAUSAL ESTIMANDS, AND OBSERVED DATA

Consider an experiment with N units, a binary treatment W, and a binary outcome Y. Under the Stable Unit Treatment Value Assumption (Rubin, 1980), we define $Y_i(w)$ as the potential outcome of unit i under treatment w, with $w = 1$ for treatment and $w = 0$ for control, respectively. Therefore, the potential outcomes form an $N \times 2$ matrix $\{(Y_i(1), Y_i(0))\}_{i=1}^{N}$, which is sometimes referred to as the "Science" (Rubin, 2005). With a binary outcome, there are only four types of individuals possible, defined by the pair $(Y_i(1), Y_i(0))$ of potential outcomes. In particular, if we imagine Y being a binary outcome of survival status, $(Y_i(1), Y_i(0)) = (1, 1)$ would be those who always survived, $(Y_i(1), Y_i(0)) = (0, 0)$ would never survive regardless of treatment, and so forth.

TABLE 1 The summarized Science Table

	$Y(1) = 1$	$Y(1) = 0$	row sum
$Y(0) = 1$	N_{11}	N_{01}	$S = N_{11} + N_{01}$
$Y(0) = 0$	N_{10}	N_{00}	$N - S$

TABLE 2 The observed Data

	$Y^{\text{obs}} = 1$	$Y^{\text{obs}} = 0$	row sum
$W = 1$	n_{11}^{obs}	n_{10}^{obs}	N_1
$W = 0$	n_{01}^{obs}	n_{00}^{obs}	N_0

The treatment has a positive impact for those with $(Y_i(1), Y_i(0)) = (1, 0)$ and a negative impact for those with $(Y_i(1), Y_i(0)) = (0, 1)$. Because there are only four types of units, the full $N \times 2$ Science Table can be summarized by a 2×2 table formed by the cell counts $N_{jk} = \#\{i : Y_i(1) = j, Y_i(0) = k\}$ for j and $k = 0, 1$ (see Table 1).

Causal effects are defined as comparisons between the potential outcomes. On the difference scale, $\tau_i = Y_i(1) - Y_i(0)$ is the individual-level causal effect for unit i. Define $p_w = \sum_{i=1}^{N} Y_i(w)/N = \bar{Y}(w)$ as the proportion of the potential outcome $Y_i(w)$ being 1. Then, the average causal effect is defined as

$$\tau = \frac{1}{N} \sum_{i=1}^{N} \tau_i = p_1 - p_0 = \frac{N_{10} - N_{01}}{N}.$$

We focus on τ. It is conceptually straightforward to extend our discussion to other causal measures (Ding & Dasgupta, 2016; Robins, 1988).

Consider a completely randomized experiment with N_1 units receiving treatment and N_0 control. The observed outcomes are deterministic functions of the treatment assignment and potential outcomes, that is, $Y_i^{\text{obs}} = W_i Y_i(1) + (1 - W_i) Y_i(0)$. Because both the treatment assignments and observed outcomes are binary, there are four observed types of the units classified by (W_i, Y_i^{obs}), which gives a different 2×2 table formed by the cell counts $n_{wy}^{\text{obs}} = \#\{i : W_i = w, Y_i^{\text{obs}} = y\}$ for $w = 0, 1$ and $y = 0, 1$ (see Table 2). This Table is distinct from the unknown Science Table 1. Importantly, the potential outcomes, the cell counts N_{jk}'s, and the causal estimand τ are all fixed. The observed cell counts n_{wy}^{obs}'s, however, are random, but the randomness comes solely from the physical randomization of the treatment assignment.

3 | INFERENCE UNDER MONOTONICITY

We first discuss an important simplifying case where the potential outcomes satisfy monotonicity.

Assumption 1. (Monotonicity) $Y_i(1) \geq Y_i(0)$ for each unit i.

Monotonicity means that treatment is not harmful to any unit, which rules out the existence of potentially harmed units with $(Y_i(1), Y_i(0)) = (0, 1)$, making $N_{01} = 0$. The case with $Y_i(1) \leq Y_i(0)$ for all i is analogous. Monotonicity is not refutable based on the observed data as long as the treatment is not harmful to the outcome on average. Monotonicity is a strong assumption: It imposes a maximal correlation between the potential outcomes $Y(1)$ and $Y(0)$ and guarantees the identifiability of all the cell counts N_{jk}'s, as described by Proposition 1.

Proposition 1. *Under monotonicity, $N_{01} = 0$, and we can identify (i.e., express parameters as expectations of observed data) the N_{jk}'s by*

$$N_{11} = E\left(\frac{N}{N_0}n_{01}^{obs}\right), \quad N_{00} = E\left(\frac{N}{N_1}n_{10}^{obs}\right), \quad N_{10} = E\left(N - \frac{N}{N_0}n_{01}^{obs} - \frac{N}{N_1}n_{10}^{obs}\right).$$

Proposition 1 immediately results in unbiased moment estimators for the N_{jk}'s made by plugging in sample moments. In particular, $\widehat{N}_{10} = N - (N/N_0)n_{01}^{obs} - (N/N_1)n_{10}^{obs}$ and

$$\widehat{\tau} = \frac{\widehat{N}_{10}}{N} = 1 - \frac{n_{01}^{obs}}{N_0} - \frac{n_{10}^{obs}}{N_1} = \frac{n_{11}^{obs}}{N_1} - \frac{n_{01}^{obs}}{N_0} \equiv \widehat{p}_1 - \widehat{p}_0,$$

where $\widehat{p}_1$ and $\widehat{p}_0$ are the observed proportions of the outcomes being one under treatment and control, respectively. Monotonicity also allows for estimation of the correlation of potential outcomes, giving the following extension of Neyman (1923).

Proposition 2. *Suppose Assumption 1 holds. The randomization distribution of $\widehat{\tau}$ has mean τ and variance*

$$\text{var}(\widehat{\tau}) = \frac{N}{N-1}\left\{\frac{p_1(1-p_1)}{N_1} + \frac{p_0(1-p_0)}{N_0} - \frac{\tau(1-\tau)}{N}\right\}. \tag{1}$$

The variance can be estimated by plugging in

$$\widehat{V} = \frac{N}{N-1}\left\{\frac{\widehat{p}_1(1-\widehat{p}_1)}{N_1} + \frac{\widehat{p}_0(1-\widehat{p}_0)}{N_0} - \frac{\widehat{\tau}(1-\widehat{\tau})}{N}\right\}. \tag{2}$$

Furthermore, $(\widehat{\tau} - \tau)/\widehat{V}^{1/2} \to \mathcal{N}(0,1)$ in distribution.

Unlike the classic Neyman (1923) variance expression, all terms in expression (1) are identifiable. Although a moment estimator with an explicit form such as this can be useful to illustrate sources of information, it might not make full use of the information and can sometimes give estimates outside of the parameter space. An alternative approach is to utilize likelihood and Bayesian inferences for the parameters of interest, which restricts our attention to only those values that are possible. Now, because $\{(Y_i(1), Y_i(0))\}_{i=1}^N$ are fixed numbers, we cannot write down the likelihood function based on the usual binomial models. We can, however, write it down according to an urn model induced by the completely randomized experiment. In particular, view the finite population as a fixed urn containing three types of balls corresponding to the three types of units defined by $(Y(1), Y(0)) = (1, 1), (1, 0)$, and $(0, 0)$. We have N_{11} balls of type $(1, 1)$, N_{10} balls of type $(1, 0)$, and $N - N_{11} - N_{10}$ balls of type $(0, 0)$. We can thus parameterize the population with only N_{11} and N_{10}. A completely randomized experiment is then equivalent to drawing N_1 balls from this urn to form the treatment arm and using the remaining N_0 balls to form the control arm. This allows for writing down the likelihood based on the observed data as a multivariate hypergeometric distribution as given in Theorem 1 below.

Theorem 1. *Under monotonicity, the likelihood function of (N_{10}, N_{11}) is*

$$\binom{N_{11}}{N_{11} - n_{01}^{obs}}\binom{N_{10}}{n_{11}^{obs} + n_{01}^{obs} - N_{11}}\binom{N - N_{10} - N_{11}}{n_{10}^{obs}} \bigg/ \binom{N}{N_1},$$

for any (N_{10}, N_{11}) in the region

$$\left\{(N_{10}, N_{11}) : n_{01}^{obs} \le N_{11} \le n_{11}^{obs} + n_{01}^{obs} \le N_{10} + N_{11} \le N - n_{10}^{obs}\right\}. \tag{3}$$

The likelihood is zero elsewhere.

There are several curious aspects and consequences to this theorem, which we now discuss. First, before obtaining data, the condition $N_{10} + N_{11} + N_{00} = N$ restricts (N_{10}, N_{11}) to take $(N + 2)(N + 1)/2$ possible values, and τ can take values k/N for any integer $k \in [-N, N]$. After observing the data, (N_{10}, N_{11}) can take only $(n_{11}^{\text{obs}} + 1)(n_{00}^{\text{obs}} + 1) < (N + 2)(N + 1)/2$ possible values due to (3), and there are, at most, $n_{11}^{\text{obs}} + n_{00}^{\text{obs}} + 1$ possible values for τ, a fact noticed by Rigdon and Hudgens (2015a) from a different perspective.

Second, there are no modeling assumptions on the outcomes. The likelihood is completely driven by the physical randomization. This idea is not entirely new: Such an urn model was used in Neyman's (1923) seminal causal inference paper for deriving the unbiased moment estimator and confidence interval for τ.

Third, Theorem 1 allows for a maximum likelihood estimate of τ, obtained by maximizing the likelihood over all possible (N_{10}, N_{11}) values. This likelihood function can also play a central role in model-free Bayesian inference. For example, if we put a uniform prior on the $(N + 2)(N + 1)/2$ feasible points of (N_{10}, N_{11}), the posterior distribution of (N_{10}, N_{11}) concentrates only on the $(n_{11}^{\text{obs}} + 1)(n_{00}^{\text{obs}} + 1)$ points within region (3) and is proportional to the likelihood. If we have prior information other than the uniform distribution, we could also incorporate it into our Bayesian inference. Based on the posterior distribution of (N_{10}, N_{11}), it is straightforward to obtain the posterior distribution of τ.

4 | INFERENCE WITHOUT MONOTONICITY

We next relax the monotonicity assumption. Without monotonicity, the unknown parameters in the Science Table, $(N_{11}, N_{10}, N_{01}, N_{00})$, are no longer identifiable by the observed data. This introduces an additional complication from before, but the overall intuition is the same. Without identifiability of $(N_{11}, N_{10}, N_{01}, N_{00})$, the sampling variance of $\hat{\tau}$ cannot be identified by the observed data, the likelihood function will be flat over a region with multiple points, and Bayesian inference will be strongly driven by the prior distribution. We can, however, weaken monotonicity in such a way that preserves identifiability in a sensitivity analysis approach. This can also be used to generate estimation regions rather than point estimates. Finally, this approach also allows for continued use of the likelihood approach discussed above.

The key insight is that, for a known N_{01}, all the cell counts of N_{jk}'s are identifiable, allowing us to parameterize our urn model with (N_{10}, N_{11}) as before. We therefore choose N_{01} as the sensitivity parameter, with $N_{01} = 0$ corresponding to monotonicity.

We first present some extensions of the previous propositions and then discuss how to use them for this sensitivity analysis approach to variance estimation. We then extend the likelihood and Bayesian inference procedures from before.

Proposition 3. *When N_{01} is known, we can identify the N_{jk}'s by*

$$N_{11} = E\left(\frac{N}{N_0} n_{01}^{\text{obs}} - N_{01}\right), \ N_{00} = E\left(\frac{N}{N_1} n_{10}^{\text{obs}} - N_{01}\right), \ N_{10} = E\left(N + N_{01} - \frac{N}{N_0} n_{01}^{\text{obs}} - \frac{N}{N_1} n_{10}^{\text{obs}}\right).$$

The above derives from the marginal distributions of the potential outcomes, which imposes weak restrictions on their association, captured by bounds on N_{01}, given the data being binary.

Proposition 4. *The number of potentially harmed units, N_{01}, is bounded by*

$$\max(0, -N\tau) \leq N_{01} \leq \min \{N p_0, N(1 - p_1)\}. \tag{4}$$

The bounds in (4) are the Frechét–Hoeffding bounds (cf. Nelsen, 2007) for N_{01} based on the marginal distributions of the potential outcomes.

In many realistic cases, it seems plausible to assume a nonnegative correlation between the potential outcomes.

Assumption 2. (Nonnegatively correlated potential outcomes) The potential outcomes are uncorrelated, that is, $\{Y_i(1)\}_{i=1}^N$ and $\{Y_i(0)\}_{i=1}^N$ have nonnegative finite population covariance

$$S_{10} = \frac{1}{N-1} \sum_{i=1}^N \left\{ Y_i(1) - \bar{Y}(1) \right\} \left\{ Y_i(0) - \bar{Y}(0) \right\} \geq 0.$$

It seems implausible that the potential outcomes $\{Y_i(1), Y_i(0)\}$ for unit i are negatively correlated because they are about the same aspect of the same unit. Under a superpopulation model, Assumption 2 could be justified if the individual potential outcomes are driven by the same latent factor in the same direction. For example, Assumption 2 holds if $Y_i(w) = f_w(U_i, \varepsilon_{iw})$, where U_i is a variable representing unit i's characteristic, $\{f_1(u, e), f_0(u, e)\}$ are two monotone functions in u, and the ε_{iw}'s are independent errors.

Proposition 5. *Under Assumption 2,* $\max(0, -N\tau) \leq N_{01} \leq Np_0(1-p_1)$; *if we further assume a nonnegative average causal effect* $\tau \geq 0$, *then*

$$0 \leq N_{01} \leq Np_0(1-p_1). \tag{5}$$

Without loss of generality, we assume that our data have $\hat{\tau} > 0$, and therefore, we either assume monotonicity or conduct sensitivity analysis within the empirical range of (5).

Proposition 6. *With a known N_{01}, the variance of $\hat{\tau}$ is*

$$\mathrm{var}(\hat{\tau}) = \frac{N}{N-1} \left\{ \frac{p_1(1-p_1)}{N_1} + \frac{p_0(1-p_0)}{N_0} - \frac{\tau(1-\tau)}{N} - \frac{2N_{01}}{N^2} \right\}. \tag{6}$$

The bounds of the above variance over the possible values of N_{01} as delineated by region (5) are

$$\frac{N}{N-1} \left\{ \frac{\frac{N_0}{N} p_1(1-p_1)}{N_1} + \frac{\frac{N_1}{N} p_0(1-p_0)}{N_0} \right\} \leq \mathrm{var}(\hat{\tau})$$

$$\leq \frac{N}{N-1} \left\{ \frac{p_1(1-p_1)}{N_1} + \frac{p_0(1-p_0)}{N_0} - \frac{\tau(1-\tau)}{N} \right\}.$$

In Proposition 6, the upper bound of $\mathrm{var}(\hat{\tau})$ corresponds to monotonicity with $N_{01} = 0$, and the lower bound corresponds to uncorrelated potential outcomes with $S_{10} = 0$.

4.1 | Variance estimation in a sensitivity analysis

Although τ depends only on the marginal distributions of the potential outcomes, the variance of $\hat{\tau}$ depends further on the correlation between the potential outcomes. Ding and Dasgupta (2016) showed that (1) is an upper bound for the true sampling variance of $\hat{\tau}$ without monotonicity. However, this result does not show explicitly the impact of the correlation between the potential outcomes on the variability of the estimator for τ. Proposition 6 does. In particular, we can conduct a sensitivity analysis by varying N_{01} within (5) to get a series of variance estimators according to (6). If we believe that N_{01} is in a specific range, we can take the maximum and minimum of the

variances as a range of possible uncertainty estimates. Generally, as N_{01} increases, the variance goes down; the most conservative (largest) variance estimate corresponds to monotonicity.

To close this subsection, it is worth commenting on an exact interval for τ by inverting randomization tests for all Science Tables (Rigdon & Hudgens, 2015b). To achieve finite-sample exactness, Rigdon and Hudgens' (2015b) confidence interval will often be wider than the Wald interval corresponding to the most conservative variance estimate under monotonicity. We do not claim to replace Rigdon and Hudgens' (2015b) confidence interval with the sequence of variance estimators obtained from (6), because our analysis is fundamentally asymptotic. Our focus is sensitivity analysis, which can also be adapted into Rigdon and Hudgens' (2015b) strategy. In particular, we can construct exact confidence intervals for τ by inverting randomization tests for fixed values of the sensitivity parameter N_{01}. Rigdon and Hudgens' (2015b) confidence interval is the union of these confidence intervals under all possible values of N_{01}.

4.2 | Likelihood and Bayesian inferences

The discussion above allows for getting sharper estimates on the variance of the classic moment estimators, as compared to the classic Neyman approach. We can also extend the likelihood approach shown for monotonicity in a similar fashion to obtain estimators restricted to the support of the parameter space. For a fixed N_{01}, the likelihood function, based on an urn model with four types of balls, is given by the following theorem.

Theorem 2. *Given a fixed N_{01}, the likelihood function for (N_{10}, N_{11}) is*

$$\sum_{x \in \mathscr{F}} \binom{N_{11}}{x} \binom{N_{10}}{n_{11}^{\text{obs}} - x} \binom{N_{01}}{N_{01} + N_{11} - n_{01}^{\text{obs}} - x} \binom{N - N_{11} - N_{10} - N_{01}}{n_{10}^{\text{obs}} + n_{01}^{\text{obs}} + x - N_{01} - N_{11}} \Bigg/ \binom{N}{N_1}, \quad (7)$$

where the feasible region of the above summation is $\mathscr{F} = \{x : L \leq x \leq U\}$ with

$$L = \max\left(0, n_{11}^{\text{obs}} - N_{10}, N_{11} - n_{01}^{\text{obs}}, N_{01} + N_{11} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}\right),$$
$$U = \min\left(N_{11}, n_{11}^{\text{obs}}, N_{01} + N_{11} - n_{01}^{\text{obs}}, N - N_{10} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}\right).$$

Note that the x in the sum in (7) represents the number of "always survivors" randomized to the treatment group; the formula marginalizes over this to get the overall likelihood. When $N_{01} = 0$, the feasible region of x collapses to the point $x = N_{11} - n_{01}^{\text{obs}}$, and the likelihood function in Theorem 2 reduces to the one in Theorem 1. The proof of Theorem 2 in the Appendix shows that, for fixed $0 \leq N_{01} \leq N\hat{p}_0(1 - \hat{p}_1)$, the likelihood is zero outside the following region of (N_{10}, N_{11}):

$$\max\left(0, n_{01}^{\text{obs}} - N_{01}\right) \leq N_{11} \leq \min\left(n_{01}^{\text{obs}} + n_{11}^{\text{obs}}, N - n_{00}^{\text{obs}} - N_{01}\right),$$
$$0 \leq N_{10} \leq N - n_{01}^{\text{obs}} - n_{10}^{\text{obs}}, \quad (8)$$
$$\max\left(n_{11}^{\text{obs}} + n_{01}^{\text{obs}} - N_{01}, n_{11}^{\text{obs}}\right) \leq N_{10} + N_{11} \leq N - n_{10}^{\text{obs}}.$$

We can then do a sensitivity analysis to see how the likelihood function and the maximum likelihood estimator change as we increase N_{01}. These curves can also be calculated for any estimand of interest as the population is fully specified by (N_{11}, N_{10}), given N_{01}. For Bayesian inference, if we impose a uniform prior on (N_{10}, N_{11}), the posterior distribution of (N_{10}, N_{11}) is proportional to (7). This immediately gives posterior distributions of τ.

Copas (1973) treated (7) as a likelihood function for (N_{11}, N_{10}, N_{01}) and observed its pathological behaviors due to the unidentifiability issue. An alternative Bayesian approach might impose a prior distribution on the sensitivity parameter N_{01}. Regardless of the identifiability issue, the

posterior distributions of the parameters of interest will always be proper because of finite support. Watson (2014) gave a detailed discussion on Bayesian inference by imposing prior distributions on (N_{11}, N_{10}, N_{01}) and making connections to posterior predictive checks (Rubin, 1984, 1998). However, inference might then be driven by the prior distribution of N_{01}, an unidentifiable parameter from the data. Therefore, we recommend the sensitivity analysis approach in both likelihood and Bayesian inferences to explicitly show the impact of the correlation between potential outcomes.

5 | THE ATTRIBUTABLE EFFECT AND THE TREATMENT EFFECT ON THE TREATED

In the previous sections, we focused on the average treatment effect, which is a fixed parameter depending only on the Science Table. In practice, other causal quantities may be of scientific interest. For instance, Rosenbaum (2001) proposed to estimate the effect attributable to the treatment, that is,

$$A = \sum_{i=1}^{N} W_i \tau_i,$$

which is closely related to the average treatment effect on the treated units $\tau^W = \sum_{i=1}^{N} W_i \tau_i / N_1 = A/N_1$. Because the difference between A and τ^W is the fixed scaling factor N_1, we discuss only the inference of the attributable effect A. Both causal quantities A and τ^W depend on the treatment assignment as well as the Science Table, and thus, they are themselves random variables. Therefore, as Rosenbaum (2001) suggested, we need to extend the traditional concepts of point and interval estimation to point and interval prediction of random variables in frequentists' inference. Rosenbaum (2001) proposed a Hodges–Lehmann-type prediction interval for A by inverting a sequence of randomization tests. We extend Rosenbaum's (2001) discussion in the Appendix and focus on Neyman-type and Bayesian inferences for A in the main text below.

5.1 | Neyman-type repeated-sampling evaluation

A natural estimator for A is $N_1 \hat{\tau}$. The following proposition shows that $N_1 \hat{\tau}$ is an unbiased predictor of A, and the mean squared error for this prediction depends only on the marginal distribution of $Y(0)$.

Proposition 7. *Over all possible randomizations, $E(A - N_1 \hat{\tau}) = 0$ and*

$$\text{var}(A - N_1 \hat{\tau}) = \frac{NN_1}{N_0} S_0^2 = \frac{N^2 N_1}{N_0 (N - 1)} p_0 (1 - p_0), \tag{9}$$

where $S_0^2 = (N - 1)^{-1} \sum_{i=1}^{N} \{Y_i(0) - \bar{Y}(0)\}^2 = N p_0 (1 - p_0)/(N - 1)$ is the finite population variance of the control potential outcome. Therefore, A can be unbiasedly predicted by $N_1 \hat{\tau}$ with estimated mean squared error $N^2 N_1 \hat{p}_0 (1 - \hat{p}_0)/\{N_0 (N - 1)\}$.

Based on Proposition 7 and a normal approximation, we can use $N_1 \hat{\tau} \pm \Phi^{-1}(1 - \alpha/2) \times \sqrt{N^2 N_1 \hat{p}_0 (1 - \hat{p}_0)/\{N_0 (N - 1)\}}$ as a $1 - \alpha$ asymptotic prediction interval of A, where $\Phi^{-1}(1 - \alpha/2)$ is the upper $1 - \alpha/2$ quantile of the standard normal distribution. Proposition 7 does not rely on monotonicity. Moreover, the first identity in (9) also holds for general outcomes. Interestingly, the variance formula (9) does not depend on the correlation between the potential outcomes, which was hinted at by Robins (1988) and Hansen and Bowers (2009). In particular, by allowing the

target of estimation to vary in a randomized experiment, one can seemingly avoid the unidentifiable issue, but the resulting analysis is then conditional, in some sense, on the realized assignment.

5.2 | Bayesian inference

As shown in (A1) in the proof of Theorem 2 in the Appendix, the attributable effect can be written as

$$A = n_{11}^{obs} + n_{01}^{obs} - N_{01} - N_{11} = n_{11}^{obs} + n_{01}^{obs} - S, \tag{10}$$

with $S = N_{01} + N_{11}$ defined in Table 1. Note that S is a parameter depending on the Science Table 1. Formula (10) shows a linear relationship between A and S, which makes the statistical inference of A simpler via the statistical inference of S. Based on (10), the posterior distribution of $S = N_{01} + N_{11}$ determines the posterior distribution of A. Therefore, with fixed N_{01} (zero under monotonicity and positive for a sensitivity analysis), obtaining the posterior distribution of A is straightforward once we obtain the posterior distribution of N_{11}.

6 | ILLUSTRATION

We reanalyze the data in Rosenbaum (2002, p. 191) concerning death in the London underground. In the London underground, some train stations have a drainage pit below the tracks. When an "incident" happens (i.e., a passenger falls, jumps, or is pushed from the station platform), such a pit is a place to escape contact with the wheels of the train. Researchers are interested in the mortality in stations with and without such a pit. In stations without a pit, only 5 lived out of 21 recorded "incidents." For "incidents" in stations with a pit, 18 out of 32 lived. Therefore, the observed data can be summarized by $(n_{11}^{obs}, n_{10}^{obs}, n_{01}^{obs}, n_{00}^{obs}) = (18, 14, 5, 16)$, viewing "pit" versus "no pit" as treatment versus control, and life as the outcome. For illustration, we view this data set as from a hypothetical completely randomized experiment, ignoring any issues of confounding.

Under monotonicity, the moment-based estimator is $\hat{\tau} = 0.324$, that is, we estimate that the chance of survival is about 32 percentage points higher for stations with a pit. Using the variance estimator in (2), we end up with a confidence interval of $[0.106, 0.543]$, which is 13% narrower than Neyman's (1923) confidence interval of $[0.072, 0.577]$ (see the first row of Table 3).

We then conduct a sensitivity analysis on monotonicity by varying the value of N_{01}, where $N_{01} = 0$ corresponds to monotonicity, $N_{01} = 5$ corresponds to uncorrelated potential outcomes, and $N_{01} = 2$ is a value between these two extreme cases. Rows 2 and 3 of Table 3 show estimates and associated confidence intervals for these two different values of N_{01}. They are smaller. If we believe some would be harmed, we are then more certain of the average causal effect. Our improved variance estimator (2) and the Bayesian approach (with a uniform prior and thus

TABLE 3 Moment and Bayes estimators with $(n_{11}^{obs}, n_{10}^{obs}, n_{01}^{obs}, n_{00}^{obs}) = (18, 14, 5, 16)$.
Columns 2–4 show the point estimator, the interval estimator, and its length

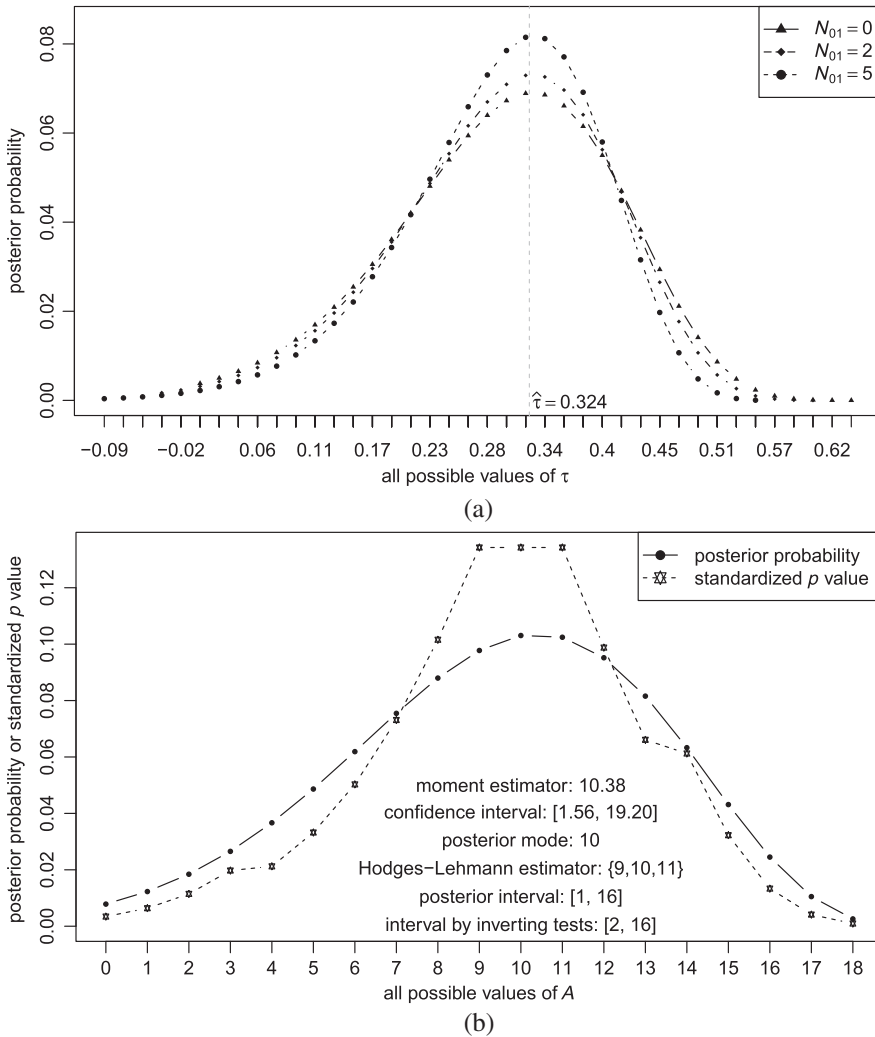| N_{01}	Neyman's variance	Improved variance	Bayes
0	0.324 [0.072, 0.577] 0.505	0.324 [0.106, 0.543] 0.437	0.301 [0.075, 0.509] 0.434
2	same as above	0.324 [0.119, 0.530] 0.411	0.301 [0.075, 0.490] 0.415
5	same as above	0.324 [0.141, 0.508] 0.367	0.301 [0.094, 0.472] 0.378

FIGURE 1 Example with observed data $(n_{11}^{obs}, n_{10}^{obs}, n_{01}^{obs}, n_{00}^{obs}) = (18, 14, 5, 16)$. (a) Sensitivity analysis for the posterior distribution of τ. Three posterior distributions of τ correspond to three values of the sensitivity parameter N_{01}. (b) Attributable effect under monotonicity. The p values are standardized to have summation 1, in order to compare with the posterior distribution

posterior proportional to the likelihood (7)) both provide improved inference. The moment estimator is close to the Bayesian posterior modes, but there is slight shift of 2 percentage points.

Figure 1a shows the posterior distributions of τ with $N_{01} = 0, 2$ and 5. The posterior distribution has a higher peak and lighter tails with larger N_{01}. This conforms to the frequentists' property that the variance of $\hat{\tau}$ becomes larger when N_{01} gets smaller, with monotonicity being the extreme case.

Regarding the attributable effect under monotonicity, the Hodges–Lehmann-type estimator is 9, 10, or 11, and the 95% interval estimate is [2, 16], based on Rosenbaum (2001). The posterior mode for A is 10, and the 95% highest probability interval for A is [1, 16]. Figure 1b compares the posterior probabilities and standardized p values for testing $A = a$, showing that they have similar shapes. The moment estimator for A is 10.38 with confidence interval [1.56, 19.20]. The

moment estimator is outside of the range of the parameter because A must be an integer. Worse, the associated interval estimate is wider, with an upper limit larger than $n_{11}^{\mathrm{obs}} = 18$, the maximum possible value of A under monotonicity due to $A = n_{11}^{\mathrm{obs}} + n_{01}^{\mathrm{obs}} - N_{11} \leq n_{11}^{\mathrm{obs}}$.

7 | CONCLUSION

For binary experimental data, we proposed several model-free inferential procedures for the average treatment effect and the attributable effect. We believe demonstrating that likelihood and Bayesian estimation without modeling is possible is a worthwhile proof of concept for an alternate form of thinking about estimation when the assignment mechanism is known. For further connections and comparisons, see Greenland (1991), Ding (2017), Chiba (2015), and Ding and Dasgupta (2016).

Some researchers have proposed randomization-based procedures for causal effects with noncompliance (Imbens & Rosenbaum, 2005; Keele, Small, & Grieve, 2017; Rubin, 1998), with general intermediate variables (Nolen & Hudgens, 2011), and with interference (Rigdon & Hudgens, 2015a; Rosenbaum, 2012). It is our ongoing work to extend the current approaches to these settings.

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REFERENCES

Agresti, A. (2013). *Categorical data analysis* (3rd ed.). Hoboken, NJ: John Wiley & Sons, Inc.

Aronow, P. M., Green, D. P., & Lee, D. K. (2014). Sharp bounds on the variance in randomized experiments. *The Annals of Statistics*, *42*, 850–871.

Chiba, Y. (2015). Exact tests for the weak causal null hypothesis on a binary out come in randomized trials. *Journal of Biometrics & Biostatistics*, *6*, 244. https://doi.org/10.4172/21556180.1000244

Copas, J. (1973). Randomization models for the matched and unmatched 2 × 2 tables. *Biometrika*, *60*, 467–476.

Ding, P. (2017). A paradox from randomization-based causal inference. *Statistical Science*, *32*, 331–345.

Ding, P., & Dasgupta, T. (2016). A potential tale of two-by-two tables from completely randomized experiments. *Journal of the American Statistical Association*, *111*, 157–168.

Fisher, R. A. (1935). *The design of experiments* (1st ed.). Edinburgh, UK: Oliver & Boyd.

Fogarty, C. B., Mikkelsen, M. E., Gaieski, D. F., & Small, D. S. (2016). Discrete optimization for interpretable study populations and randomization inference in an observational study of severe sepsis mortality. *Journal of the American Statistical Association*, *111*, 447–458.

Greenland, S. (1991). On the logical justification of conditional tests for two-by-two contingency tables. *The American Statistician*, *45*, 248–251.

Hansen, B. B., & Bowers, J. (2009). Attributing effects to a cluster-randomized get-out-the-vote campaign. *Journal of the American Statistical Association*, *104*, 873–885.

Hodges, J. J. L., & Lehmann, E. L. (1963). Estimates of location based on rank tests. *The Annals of Mathematical Statistics*, *34*, 598–611.

Imbens, G. W., & Rosenbaum, P. R. (2005). Robust, accurate confidence intervals with a weak instrument: Quarter of birth and education. *Journal of the Royal Statistical Society: Series A (Statistics in Society)*, *168*, 109–126.

Imbens, G. W., & Rubin, D. B. (2015). *Causal inference in statistics, social, and biomedical sciences: An introduction*. New York, NY: Cambridge University Press.

Keele, L., Small, D., & Grieve, R. (2017). Randomization-based instrumental variables methods for binary outcomes with an application to the ʹIMPROVEʹ trial. *Journal of the Royal Statistical Society: Series A (Statistics in Society)*, *180*, 569–586.

Li, X., & Ding, P. (2016). Exact confidence intervals for the average causal effect on a binary outcome. *Statistics in Medicine*, *35*, 957–960.

Li, X., & Ding, P. (2017). General forms of finite population central limit theorems with applications to causal inference. *Journal of the American Statistical Association*, *112*, 1759–1769.

Nelsen, R. B. (2007). *An introduction to copulas* (2nd ed.). New York, NY: Springer.

Neyman, J. (1923). On the application of probability theory to agricultural experiments. Essay on principles. Section 9. *Statistical Science*, *5*, 465–472.

Nolen, T. L., & Hudgens, M. G. (2011). Randomization-based inference within principal strata. *Journal of the American Statistical Association*, *106*, 581–593.

Rigdon, J., & Hudgens, M. G. (2015a). Exact confidence intervals in the presence of interference. *Statistics and Probability Letters*, *105*, 130–135.

Rigdon, J., & Hudgens, M. G. (2015b). Randomization inference for treatment effects on a binary outcome. *Statistics in Medicine*, *34*, 924–935.

Robins, J. M. (1988). Confidence intervals for causal parameters. *Statistics in Medicine*, *7*, 773–785.

Rosenbaum, P. R. (1999). Reduced sensitivity to hidden bias at upper quantiles in observational studies with dilated treatment effects. *Biometrics*, *55*, 560–564.

Rosenbaum, P. R. (2001). Effects attributable to treatment: Inference in experiments and observational studies with a discrete pivot. *Biometrika*, *88*, 219–231.

Rosenbaum, P. R. (2002). *Observational studies* (2nd ed.). New York, NY: Springer.

Rosenbaum, P. R. (2012). Interference between units in randomized experiments. *Journal of the American Statistical Association*, *102*, 191–200.

Rubin, D. B. (1974). Estimating causal effects of treatments in randomized and nonrandomized studies. *Journal of Educational Psychology*, *66*, 688–701.

Rubin, D. B. (1980). Comment on "Randomization analysis of experimental data: The Fisher randomization test". *Journal of the American Statistical Association*, *75*, 591–593.

Rubin, D. B. (1984). Bayesianly justifiable and relevant frequency calculations for the applied statistician. *The Annals of Statistics*, *12*, 1151–1172.

Rubin, D. B. (1998). More powerful randomization-based *p*-values in double-blind trials with non-compliance. *Statistics in Medicine*, *17*, 371–385.

Rubin, D. B. (2005). Causal inference using potential outcomes: Design, modeling, and decisions. *Journal of the American Statistical Association*, *100*, 322–331.

Wang, W. (2015). Exact optimal confidence intervals for hypergeometric parameters. *Journal of the American Statistical Association*, *110*, 1491–1499.

Watson, D. A. (2014). *Complications in causal inference: Incorporating information observed after treatment is assigned* (PhD thesis). Harvard University, Cambridge, MA.

SUPPORTING INFORMATION

Additional supporting information may be found online in the Supporting Information section at the end of the article.

APPENDIX

Proofs of the theorems

The proofs of the propositions are relatively straightforward, and we relegate them to the supplementary material. Here, we give the proofs for the theorems.

Proof of Theorem 1. Under monotonicity, the units with $(W_i, Y_i^{obs}) = (1, 1)$ are $(1, 1)$ or $(1, 0)$ units, the units with $(W_i, Y_i^{obs}) = (1, 0)$ are all $(0, 0)$ units, the units with $(W_i, Y_i^{obs}) = (0, 1)$ are all $(1, 1)$ units, and the units with $(W_i, Y_i^{obs}) = (0, 0)$ are $(0, 0)$ or $(1, 0)$ units. Define $N_{bc,w}$ as the number of (b, c) units within observed treatment group w $(b, c, w = 0, 1)$. Then, the observed data allow us to obtain

$$N_{11,1} = N_{11} - n_{01}^{obs}, \qquad\qquad N_{11,0} = n_{01}^{obs},$$
$$N_{00,1} = n_{10}^{obs}, \qquad\qquad N_{00,0} = N_{00} - n_{10}^{obs},$$
$$N_{10,1} = n_{11}^{obs} - N_{11,1} = n_{11}^{obs} + n_{01}^{obs} - N_{11}, \qquad N_{10,0} = N_{10} - N_{10,1} = N_{10} + N_{11} - n_{11}^{obs} - n_{01}^{obs}.$$

The above shows that we know the number of each type of unit in both treatment arms, based on the observed counts and the totals N_{bc}. Because all the counts are nonnegative integers, we have the following restriction on (N_{10}, N_{11}):

$$n_{01}^{obs} \le N_{11} \le n_{11}^{obs} + n_{01}^{obs} \le N_{10} + N_{11} \le N - n_{10}^{obs}.$$

We can count that there are $(n_{11}^{obs} + 1)(n_{00}^{obs} + 1)$ possible values for (N_{10}, N_{11}) and $(n_{11}^{obs} + n_{00}^{obs} + 1)$ possible values for τ.

The completely randomized experiment corresponds to an urn model. We have an urn with N_{11} $(1, 1)$ balls, N_{10} $(1, 0)$ balls, and N_{00} $(0, 0)$ balls. The experiment is that we randomly draw N_1 balls without replacement to form the treatment arm and use the remaining balls to form the control arm. We then observe the outcomes. The above restrictions allow us to determine, based on observed data, the count vector for the three types of balls $(N_{11,1}, N_{10,1}, N_{00,1})$ that we have in the treatment arm, and similarly for control. Therefore, the probability of obtaining $(N_{11,1}, N_{10,1}, N_{00,1})$ is a multivariate hypergeometric distribution, given the values of N_{11} and N_{10}. Express this in terms of the observed data to obtain

$$\binom{N_{11}}{N_{11,1}}\binom{N_{10}}{N_{10,1}}\binom{N_{00}}{N_{00,1}} \Big/ \binom{N}{N_1} = \binom{N_{11}}{N_{11} - n_{01}^{obs}}\binom{N_{10}}{n_{11}^{obs} + n_{01}^{obs} - N_{11}}\binom{N - N_{10} - N_{11}}{n_{10}^{obs}} \Big/ \binom{N}{N_1}.$$

This is the likelihood, a function of N_{11} and N_{10}, our parameters. □

Proof of Theorem 2. Without monotonicity, the observed data classified by (W_i, Y_i^{obs}) are mixtures: The observed group $(W_i, Y_i^{obs}) = (1, 1)$ contains $(1, 1)$ and $(1, 0)$ units, the observed group $(W_i, Y_i^{obs}) = (1, 0)$ contains $(0, 1)$ and $(0, 0)$ units, the observed group $(W_i, Y_i^{obs}) = (0, 1)$

contains $(1, 1)$ and $(0, 1)$ units, and the observed group $(W_i, Y_i^{\text{obs}}) = (0, 0)$ contains $(1, 0)$ and $(0, 0)$ units. Assume that $N_{11,1} = x$, we have

$$
\begin{aligned}
N_{11,1} &= x, & N_{11,0} &= N_{11} - x, \\
N_{10,1} &= n_{11}^{\text{obs}} - x, & N_{10,0} &= N_{10} + x - n_{11}^{\text{obs}}, \\
N_{01,1} &= N_{01} + N_{11} - n_{01}^{\text{obs}} - x, & N_{01,0} &= n_{01}^{\text{obs}} + x - N_{11}, \\
N_{00,1} &= n_{10}^{\text{obs}} + n_{01}^{\text{obs}} + x - N_{01} - N_{11}, & N_{00,0} &= N - N_{10} - x - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}.
\end{aligned}
$$

As a by-product, the attributable effect is

$$
A = N_{10,1} - N_{01,1} = n_{11}^{\text{obs}} + n_{01}^{\text{obs}} - N_{01} - N_{11}. \tag{A1}
$$

The above counts must all be nonnegative, implying the following inequality on x:

$$
\begin{aligned}
\max\left(0, n_{11}^{\text{obs}} - N_{10}, N_{11} - n_{01}^{\text{obs}}, N_{01} + N_{11} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}\right) &\leq x \\
\leq \min\left(N_{11}, n_{11}^{\text{obs}}, N_{01} + N_{11} - n_{01}^{\text{obs}}, N - N_{10} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}\right).
\end{aligned} \tag{A2}
$$

When $N_{01} = 0$, the inequality collapses to $x = N_{11} - n_{01}^{\text{obs}}$, which is coherent with Theorem 1. The above inequality (A2) also imposes the following restrictions on (N_{10}, N_{11}) for a given value of N_{01} and the observed data:

$$
\begin{aligned}
0 &\leq N_{11}, & 0 &\leq n_{11}^{\text{obs}}, \\
0 &\leq N_{01} + N_{11} - n_{01}^{\text{obs}}, & 0 &\leq N - N_{10} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}, \\
n_{11}^{\text{obs}} - N_{10} &\leq N_{11}, & n_{11}^{\text{obs}} - N_{10} &\leq n_{11}^{\text{obs}}, \\
n_{11}^{\text{obs}} - N_{10} &\leq N_{01} + N_{11} - n_{01}^{\text{obs}}, & n_{11}^{\text{obs}} - N_{10} &\leq N - N_{10} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}, \\
N_{11} - n_{01}^{\text{obs}} &\leq N_{11}, & N_{11} - n_{01}^{\text{obs}} &\leq n_{11}^{\text{obs}}, \\
N_{11} - n_{01}^{\text{obs}} &\leq N_{01} + N_{11} - n_{01}^{\text{obs}}, & N_{11} - n_{01}^{\text{obs}} &\leq N - N_{10} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}, \\
N_{01} + N_{11} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}} &\leq N_{11}, & N_{01} + N_{11} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}} &\leq n_{11}^{\text{obs}}, \\
N_{01} + N_{11} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}} &\leq N_{01} + N_{11} - n_{01}^{\text{obs}}, & N_{01} + N_{11} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}} &\leq N - N_{10} - n_{10}^{\text{obs}} - n_{01}^{\text{obs}}.
\end{aligned}
$$

These inequalities can be simplied to be (8) in the main text. The inequality for N_{01} is $N_{01} \leq n_{10}^{\text{obs}} + n_{01}^{\text{obs}}$, redundant over the sensitivity analysis region $N_{01} \leq N\hat{p}_0(1 - \hat{p}_1)$, because $n_{10}^{\text{obs}} + n_{01}^{\text{obs}} \geq N\hat{p}_0(1 - \hat{p}_1)$. $\qquad \square$

Additional comments on exact inference for the attributable effect

Previously, Rigdon and Hudgens (2015b) and Li and Ding (2016) discussed the exact inference for τ, and Rosenbaum (2001) discussed the exact inference for A under monotonicity. Here, we extend Rosenbaum (2001) without assuming monotonicity.

Recall that $A = n_{11}^{\text{obs}} + n_{01}^{\text{obs}} - N_{01} - N_{11} = n_{11}^{\text{obs}} + n_{01}^{\text{obs}} - S$ in (10) and (A1). If we had a point estimator $\hat{S}$ for S, then we would have a point predictor $\hat{A} = n_{11}^{\text{obs}} + n_{01}^{\text{obs}} - \hat{S}$ for A. Furthermore, if we had an interval estimator $[\hat{S}_l, \hat{S}_u]$ for S, then we would have an interval predictor $[\hat{A}_l, \hat{A}_u]$ for A, where $\hat{A}_l = n_{11}^{\text{obs}} + n_{01}^{\text{obs}} - \hat{S}_u$ and $\hat{A}_u = n_{11}^{\text{obs}} + n_{01}^{\text{obs}} - \hat{S}_l$. We can thus separate out and capture the randomness in our target estimand with observed data, reducing the statistical uncertainty to a classic parameter estimation problem.

Randomization induces a hypergeometric distribution $n_{01}^{\text{obs}} \sim H_S$, where H_S has probability mass function $P(H_S = h) = \binom{S}{h}\binom{N-S}{N_0-h} / \binom{N}{N_0}$ for $\max(0, S - N_1) \leq h \leq \min(S, N_0)$. This hypergeometric distribution depends on the unknown parameter S, and we can thus use the number

of positive outcomes under control, n_{01}^{obs}, as our observed statistic for conducting inference on S. Fortunately, inference on S based on the hypergeometric n_{01}^{obs} is a classical statistical problem. For example, we can conduct a series of tests $H_{0s} : S = s$ and calculate the p value for each fixed s by measuring the extremeness of n_{01}^{obs} given S. A choice of the two-sided p value is

$$p(s) = \sum_{h\,:\,P(H_s=h)\leq P\left(H_s=n_{01}^{obs}\right)} P(H_s = h), \tag{A3}$$

that is, the sum of all the probability masses that are smaller than or equal to the probability mass of the observed value of the hypergeometric random variable. This effectively orders the possible values of H_s, given s, by their likelihood, and the sum in (A3) captures the total probability mass in the tails given this ordering. The Hodges–Lehmann-type point estimator for S corresponds to the s values that attain the maximum p value (Hodges & Lehmann, 1963; Rosenbaum, 2002); the point estimator may not be unique due to discreteness. The $1 - \alpha$ interval estimator contains all the s values such that $p(s) \geq \alpha$.

The choice of the two-sided p value in (A3) leads to the same procedure as in Rosenbaum (2001) and Rigdon and Hudgens (2015b). We note, however, that the classical literature on Fisher's exact test also proposed other choices of the two-sided p values based on a hypergeometric random variable (cf. Agresti, 2013, p. 92). Moreover, we could alternatively directly construct confidence intervals for S based on the hypergeometric n_{01}^{obs} without inverting tests. Please see the work of Wang (2015) for classical methods and recent developments in constructing confidence intervals for hypergeometric parameters. Overall, the relationship (10) allows for constructing different point and interval estimators for A based on different approaches for S, of which the previous approaches in Rosenbaum (2001) and Rigdon and Hudgens (2015b) are special cases. Furthermore, to make exact inference of the attributable effect, Rosenbaum (2001) invoked monotonicity, but our discussion above does not. The inference with or without monotonicity is the same.

Supplementary material for "Model-free causal inference of binary experimental data": Proofs of the Propositions

Peng Ding[*] and Luke W. Miratrix[†]

These proofs of the propositions rely on the following lemma:

Lemma 1. Assume $(c_1, \ldots, c_N)$ are constants with $\bar{c} = \sum_{i=1}^{N} c_i/N$ and $S_c^2 = \sum_{i=1}^{N}(c_i - \bar{c})^2/(N-1)$. Let $(W_1, \ldots, W_N)$ be the treatment indicators of a completely randomized experiment. We have that

$$E\left(\sum_{i=1}^{N} W_i c_i\right) = N_1 \bar{c}, \quad \mathrm{var}\left(\sum_{i=1}^{N} W_i c_i\right) = \frac{N_1 N_0}{N} S_c^2.$$

See classical survey sampling textbooks (e.g., Cochran, 1977) for the proof.

Proof of Proposition 1. Verify that

$$E(n_{01}^{\mathrm{obs}}) = E\left\{\sum_{i=1}^{N}(1 - W_i)Y_i(0)\right\} = \frac{N_0}{N} N_{11}, \quad E(n_{10}^{\mathrm{obs}}) = E\left[\sum_{i=1}^{N} W_i\{1 - Y_i(1)\}\right] = \frac{N_1}{N} N_{00}.$$

The conclusion follows. □

Proof of Proposition 2. Following Neyman (1923) (presented using modern notation in Imbens & Rubin (2015)), $\widehat{\tau}$ is unbiased for τ with variance

$$\mathrm{var}(\widehat{\tau}) = \frac{S_1^2}{N_1} + \frac{S_0^2}{N_0} - \frac{S_\tau^2}{N}, \tag{1}$$

where

$$
\begin{aligned}
S_1^2 &= \frac{1}{N-1}\sum_{i=1}^{N}\{Y_i(1) - p_1\}^2 = \frac{1}{N-1}(Np_1 - Np_1^2) = \frac{N}{N-1}p_1(1 - p_1), \\
S_0^2 &= \frac{1}{N-1}\sum_{i=1}^{N}\{Y_i(0) - p_0\}^2 = \frac{1}{N-1}(Np_0 - Np_0^2) = \frac{N}{N-1}p_0(1 - p_0), \\
S_\tau^2 &= \frac{1}{N-1}\sum_{i=1}^{N}(\tau_i - \tau)^2 = \frac{1}{N-1}\left(N_{10} - \frac{N_{10}^2}{N}\right) = \frac{N}{N-1}\tau(1 - \tau)
\end{aligned}
$$

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are the finite population variance of $Y(1), Y(0)$, and τ. For estimating the variance, note that the variance term S_τ^2 is identifiable because $N_{01} = 0$ under monotonicity, and the conclusion follows.

The consistency and asymptotic normality of $\widehat{\tau}$ follows from the finite population central limit theorem (Li & Ding, 2017). And the variance estimator can be obtained by a simple plug-in. □

Proof of Proposition 3. From Lemma 1, we have $E(\widehat{p}_1) = p_1$ and $E(\widehat{p}_0) = p_0$. Then

$$E\left(\frac{N}{N_0}n_{01}^{\mathrm{obs}} - N_{01}\right) = E(N\widehat{p}_0 - N_{01}) = Np_0 - N_{01} = (N_{01} + N_{11}) - N_{01} = N_{11},$$

$$E\left(\frac{N}{N_1}n_{10}^{\mathrm{obs}} - N_{01}\right) = E\{N(1-\widehat{p}_1) - N_{01}\} = N(1-p_1) - N_{01} = (N_{01} + N_{00}) - N_{01} = N_{00},$$

$$E\left(N + N_{01} - \frac{N}{N_0}n_{01}^{\mathrm{obs}} - \frac{N}{N_1}n_{10}^{\mathrm{obs}}\right) = N + N_{01} - Np_0 - N(1-p_1) = N_{10}.$$

□

Proof of Proposition 4. As a byproduct of the derivations in the proof of Proposition 3, we have

$$Np_0 - N_{01} \geq 0, \quad N(1-p_1) - N_{01} \geq 0, \quad N + N_{01} - Np_0 - N(1-p_1) \geq 0,$$

which further implies $\max(0, -N\tau) \leq N_{01} \leq \min\{Np_0, N(1-p_1)\}$. □

Proof of Proposition 5. Using simple algebra, we can verify that S_{10} is proportional to $N_{11}N_{00} - N_{10}N_{01}$ up to a positive constant. We also have that $N_{00} = N(1-p_1) - N_{01}$, $N_{11} = Np_0 - N_{01}$, and $N_{10} = N + N_{01} - N(1-p_1) - Np_0$, giving

$$\begin{aligned} 0 &\leq N_{11}N_{00} - N_{10}N_{01} = (Np_0 - N_{01})\{N(1-p_1) - N_{01}\} - \{N + N_{01} - N(1-p_1) - Np_0\}N_{01} \\ &= N^2 p_0(1-p_1) - NN_{01}, \end{aligned}$$

or, equivalently, $N_{01} \leq Np_0(1-p_1)$. □

Proof of Proposition 6. According to the variance formula of $\widehat{\tau}$ in (1), we need to calculate S_τ^2/N with a known N_{01}. We have

$$\frac{S_\tau^2}{N} = \frac{1}{(N-1)N}\left(\sum_{i=1}^{N}\tau_i^2 - N\tau^2\right) = \frac{1}{N-1}\left\{\frac{N_{10} + N_{01}}{N} - \left(\frac{N_{10} - N_{01}}{N}\right)^2\right\} = \frac{1}{N-1}\left(\tau - \tau^2 + \frac{2N_{01}}{N}\right),$$

and its bounds follows directly from $0 \leq N_{01} \leq Np_0(1-p_1)$. □

Proof of Proposition 7. We have $E(A) = N_1\tau = E(N_1\widehat{\tau})$, and

$$
\begin{aligned}
\mathrm{var}(A - N_1\widehat{\tau}) &= \mathrm{var}\left[\sum_{i=1}^{N} W_i\{Y_i(1) - Y_i(0)\} - \sum_{i=1}^{N} W_i Y_i(1) + \frac{N_1}{N_0}\sum_{i=1}^{N}(1 - W_i)Y_i(0)\right] \\
&= \mathrm{var}\left[\sum_{i=1}^{N} W_i\left\{Y_i(1) - Y_i(0) - Y_i(1) - \frac{N_1}{N_0}Y_i(0)\right\}\right] \\
&= \mathrm{var}\left\{\sum_{i=1}^{N} W_i Y_i(0)\cdot\frac{N}{N_0}\right\} = \frac{N^2}{N_0^2}\cdot\frac{N_1 N_0}{N(N-1)}\cdot\sum_{i=1}^{N}\{Y_i(0) - \bar{Y}(0)\}^2 \\
&= \frac{NN_1}{N_0}S_0^2 = \frac{N^2 N_1}{N_0(N-1)}p_0(1 - p_0),
\end{aligned}
$$

where the penultimate line of the proof is due to Lemma 1. $\square$

References

COCHRAN, W. G. (1977). *Sampling Techniques*. John Wiley & Sons: New York, 2nd ed.

IMBENS, G. W. & RUBIN, D. B. (2015). *Causal Inference in Statistics, Social, and Biomedical Sciences: An Introduction*. New York: Cambridge University Press.

LI, X. & DING, P. (2017). General forms of finite population central limit theorems with applications to causal inference. *Journal of the American Statistical Association* **112**, 1759–1769.

NEYMAN, J. (1923). On the application of probability theory to agricultural experiments. Essay on principles. Section 9. *Statistical Science* **5**, 465–472.