Inverse Conditional Probability Weighting with Clustered Data in Causal Inference

Zhulin He
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Abstract

Estimating the average treatment causal effect in clustered data often involves dealing with unmeasured cluster-specific confounding variables. Such variables may be correlated with the measured unit covariates and outcome. When the correlations are ignored, the causal effect estimation can be biased. By utilizing sufficient statistics, we propose an inverse conditional probability weighting (ICPW) method, which is robust to both (i) the correlation between the unmeasured cluster-specific confounding variable and the covariates and (ii) the correlation between the unmeasured cluster-specific confounding variable and the outcome. Assumptions and conditions for the ICPW method are presented. We establish the asymptotic properties of the proposed estimators. Simulation studies and a case study are presented for illustration.

Keywords: Average causal effect; Robustness; Sufficient statistic; Unmeasured cluster-specific confounding.
1 Introduction

Clustered data are usually considered as groups of units that share the same or similar characters. Some examples of clustered data are children in the classes or schools, family members in the households, and animals in the feedlots or barns. Estimating the average treatment causal effect in clustered data often involves dealing with unmeasured pre-treatment cluster-specific confounding variables, which can bring challenges in the estimation procedures. The cluster-specific confounding variables in the previous examples can be teachers’ experience and school resource (e.g., Hong and Raudenbush, 2006), neighborhood environment for the households (e.g., Brumback and Ho, 2011), and management and operations in the feedlots or barns (e.g., O’Connor et al., 2005; Ramirez et al., 2012). There are two possible reasons for why such variables are not collected into data. The first possible reason, from data collection point of view, is that it may be difficult or impossible to measure a cluster-specific confounding variable. The second possible reason, from estimation point of view, is that the cluster-specific confounding variable may be not of interest in estimation. Usually when the cluster-specific confounding variable is unobserved, its relationship to other measured variables may be unclear, which can result biased causal effect estimates. As shown in Figure 1, a dashed line or a dashed arrow represents an unclear relationship between two variables. When all three kinds of relationships with respect to unmeasured cluster-specific confounding variable are unknown, it is impossible for us to adjust for this unmeasured cluster-specific confounding variable. Therefore, additional assumptions are needed for the adjustment.

One assumption we can consider is that the treatment assignment mechanism is known. This implies the relationship between the unmeasured cluster-specific confounder and the treatment is known. Then the corresponding arrow from “cluster-specific confounder” to “treatment” in Figure 1 is not dashed anymore. Under such assumption, the inverse probability weighting (IPW) or inverse propensity score weighting, an important tool used in causal inference, can be applied to both randomized experiments and observational studies. A gen-
eral introduction of IPW method in causal inference can be found in Hernán and Robins (2018, Section 2.4). The IPW method involves estimating the probability, which is also known as a propensity score (Rosenbaum and Rubin, 1983), of a unit being applied the treatment given some information. The method has been widely studied in causal inference (e.g., Robins et al., 2000; Hirano and Imbens, 2001; Lunceford and Davidian, 2004; Cole and Hernán, 2008; VanderWeele, 2009; Ertefaie and Stephens, 2010; Tan, 2010; Sjölander et al., 2011; Zhang et al., 2012; Tchetgen Tchetgen and VanderWeele, 2012; Vansteelandt and Daniel, 2014; Imai and Ratkovic, 2014; Naimi et al., 2014; Austin and Stuart, 2015; Ogburn et al., 2015; Liu et al., 2016), missing data analysis (e.g., Little, 1986; Rotnitzky and Robins, 1995; Hogan et al., 2004; Tsiatis, 2006, Chapter 6; Chen et al., 2008; Kott and Chang, 2010; Kim and Shao, 2013, Chapter 5; Mitra and Reiter, 2011; Miao et al., 2015; Sun and Tchetgen Tchetgen, 2017; Ding and Li, 2018; Wen and Seaman, 2018), and survey statistics (e.g., Deville and Särndal, 1992; Fuller et al., 1994; Kalton and Flores-Cervantes, 2003; Kim and Im, 2014). An early case of IPW dates back to the Horvitz-Thompson estimator (Horvitz and Thompson, 1952), where the probability of a binary indicator for sampling (or missingness) is used for estimation.

The IPW method usually requires all information for estimation, which is challenging for clustered data when cluster-level information is partially or completely missing. In such case,
the assumption of no unmeasured confounder is violated. Without considering the existence of the unmeasured confounder, the method can lead researchers to the Simpson’s paradox (Simpson, 1951). This is described by Pearl et al. (2016, Section 3.6). Sensitivity analyses of the IPW estimators, when no unmeasured confounder assumption is violated, has been studied (see Brumback et al., 2004; Zhao et al., 2017).

Efforts, using the IPW method, to adjust for the unmeasured cluster-specific confounding variable have been made in recent years. Li et al. (2013) treated the unmeasured cluster-specific confounding variable as random effect and fixed effect in two propensity score models, among several models they considered, to investigate the performance of the propensity score weighting methods. As discussed by Li et al. (2013), when the number of clusters is large and the cluster size is small, fixed effect model can lead to unstable propensity score estimates due to the Neyman-Scott incidental parameter problem (Neyman and Scott, 1948). Comparatively, the random effect model does not have such problem, but it requires the independence between covariates and the unmeasured cluster-specific confounding variable. However, the independence requirement can not always be guaranteed. Yuan and Little (2007) showed biased estimation, in a missing data setting, when the outcome depends on the unmeasured cluster-specific confounding variable which may be correlated with the covariates. Skinner and D’arrigo (2011) proposed an IPW method using conditional logistic regression to overcome the bias caused by aforementioned correlations. Their method was originated from a missing data setting, then extended to binary treatment effect estimation. Later, Yang (2017) developed calibrated propensity scores for binary treatment effect estimation, which is robust to model misspecification. Other methods using propensity score with clustered data are matching (e.g., Arpino and Mealli, 2011; Arpino and Cannas, 2016; Zubizarreta and Keele, 2017) and stratification (e.g., Thoemmes and West, 2011).

In this paper, we focus on a novel method when the cluster-level confounding variable is unobserved. By utilizing the sufficient statistics, we proposed an inverse conditional probability weighting (ICPW) method, which is robust to both (i) the correlation between the
unmeasured cluster-specific confounding variable and the covariates (i.e., the left dashed line in Figure 1) and (ii) the correlation between the unmeasured cluster-specific confounding variable and the outcome (i.e., the right dashed arrow in Figure 1).

The remainder of this paper is arranged as follows. Section 2 describes clustered data structure, assumptions and models. In Section 3 we propose the ICPW method by utilizing the sufficient statistics. Asymptotic properties of the proposed estimators are shown in Section 4. Simulation studies and a case study are conducted in Section 5 and Section 6, respectively. We conclude the paper with discussion in Section 7.

2 Basic Setup

2.1 Clustered Data Structure and Estimand of Interest

Let $Y_{ij}$ be the observed outcome for the $j$th unit ($j = 1, 2, \ldots, n_i$) in the $i$th cluster ($i = 1, 2, \ldots, m$). Denote by a $p$-dimensional vector $X_{ij}$ the observed unit-specific pre-treatment covariates. Let $A_{ij}$ be the treatment variable with domain $\Omega_A$. For categorical treatments, we index treatment levels by a series of integers 0 to $K$, where $K \geq 1$. Assume there is no hidden variations of treatments, which is one component of the stable unit treatment value assumption (SUTVA) (Imbens and Rubin, 2015, Section 1.6). Denote the sample size by $n = \sum_{i=1}^{m} n_i$. For cluster-level notations, let $Y_i = (Y_{i1}, \ldots, Y_{in_i})^T$, $X_i = (X_{i1}^T, \ldots, X_{in_i}^T)^T$, and $A_i = (A_{i1}, \ldots, A_{in_i})^T$ be the $i$th cluster-level outcome, covariate, and treatment indicator, respectively. Also, let $U_i$ be a cluster-specific confounding variable summarizing unobserved information of cluster-level confounders. Assume $\Omega_U$, the domain of $U_i$, is compact.

Next, we follow the potential outcome (or called counterfactual) setup (Rubin, 1974; Neyman, 1990). Suppose each unit has two potential outcomes, $Y_{ij}(0)$ and $Y_{ij}(1)$. In particular, $Y_{ij}(0)$ is the outcome that would be realized, if the unit received control, and $Y_{ij}(1)$ is the outcome that would be realized, if the unit received treatment. Denote cluster-level potential outcomes as $Y_i(0) = (Y_{i1}(0), \ldots, Y_{in_i}(0))^T$ and $Y_i(1) = (Y_{i1}(1), \ldots, Y_{in_i}(1))^T$. 


More generally, denote cluster-level potential outcome with treatment level $a$ as $Y_i(a) = (Y_{i1}(a), \ldots, Y_{im_i}(a))^T$, where $Y_{ij}(a)$ is the unit potential outcome.

Our goal for binary treatment is to estimate the population average treatment effect, $\tau = E\{Y(1) - Y(0)\}$, which is the expectation of difference between two potential outcomes over the population. There are two ways to estimate $\tau$ without modeling potential outcomes. The first one is to calculate the unit treatment effect, namely, $Y(1) - Y(0)$, and then take the expectation with respect to the population. However, this method is not feasible due to the fundamental problem of causal inference (Rubin, 1974; Holland, 1986). Specifically, each unit can receive either treatment or control, so only one of the potential outcomes can be observed. Therefore, the unit treatment causal effect cannot be directly calculated, which implies the first way does not work. The second way for $\tau$ estimation is first taking the expectations of both potential outcomes over the population, namely, $E\{Y(0)\}$ and $E\{Y(1)\}$, and then calculating the difference of the two expectations for $\tau$. Such estimand is proposed in Rosenbaum and Rubin (1983). We also consider the latter one in the paper.

For a general notation, we are interested in estimating $E\{Y(a)\}$ and $E\{Y(a')\}$ with treatment levels $a$ and $a'$, where $a \neq a'$. Then the causal effect can be constructed as a function of $E\{Y(a)\}$ and $E\{Y(a')\}$. For example, the causal risk difference, causal relative risk, and causal odds ratio for binary outcome can be constructed as $P\{Y(a) = 1\} - P\{Y(a') = 1\}$, $P\{Y(a) = 1\}/P\{Y(a') = 1\}$, and $P\{Y(a) = 1\}/[1 - P\{Y(a) = 1\}]/P\{Y(a') = 1\}/[1 - P\{Y(a') = 1\}]$, respectively, for $a \neq a'$. In such case, we are interested in estimating $P\{Y(a) = 1\}$ and $P\{Y(a') = 1\}$.

### 2.2 Assumptions and Propensity Score for Inverse Probability Weighting

In order to identify the population average treatment effect, we consider some assumptions hold in the clustered data. Usually most assumptions in causal inference are listed in unit level. However, clustered data is different in data structure. To emphasize such difference, we consider the following assumptions (except Assumption 2) in cluster level. Besides, all assumptions (except Assumption 3) are listed with respect to binary treatment. The
corresponding general forms for non-binary treatments are given in the immediate discussion.

**Assumption 1.** \( \{ A_i, X_i, Y_i(0), Y_i(1), U_i \} \perp \perp \{ A_{i'}, X_{i'}, Y_{i'}(0), Y_{i'}(1), U_{i'} \} \) for any \( i \neq i' \). Moreover, \( A_{ij} \perp \perp A_{ij'} | X_i, U_i \) for all clusters and \( j \neq j' \).

The first component in Assumption 1 assumes all clusters are independent of each other. It satisfies the “no interference” component in the SUTVA assumption (Imbens and Rubin, 2015, Section 1.6) in cluster level. That means the treatments applied to the units in one cluster do not affect the potential outcomes of the units in any other clusters. A more general form of the first component is \( \{ A_i, X_i, \{ Y_i(a) \}_{a \in \Omega_A}, U_i \} \perp \perp \{ A_{i'}, X_{i'}, \{ Y_{i'}(a) \}_{a \in \Omega_A}, U_{i'} \} \).

The second component in Assumption 1 describes the conditional independence of the treatment assignment mechanism for units within one cluster. That is, given all information of covariates \( X_i \) and confounding variable \( U_i \) in the cluster, treatment applied to one unit does not affect that applied to other units within the same cluster.

**Assumption 2** (Consistency). \( Y_{ij} = Y_{ij}(0) I\{ A_{ij} = 0 \} + Y_{ij}(1) I\{ A_{ij} = 1 \} \), for all \( i \) and \( j \).

Assumption 2 sets up the linkage between observed outcome and potential outcomes for each unit (Hernán and Robins, 2018, Section 1.1). The meaning of this assumption is straightforward. If one unit receives control, then potential outcome \( Y_{ij}(0) \) is observed. Similarly, if one unit receives treatment, then potential outcome \( Y_{ij}(1) \) is observed. A more general description of the consistency assumption is that if \( A_{ij} = a \in \Omega_A \), then \( Y_{ij} = Y_{ij}(a) \).

**Assumption 3** (Cluster-level Positivity). The cluster-level treatment joint probability is \( P(A_i = a_i | X_i, U_i) = \prod_{j=1}^{n_i} P(A_{ij} = a_{ij} | X_i, U_i) \). It satisfies \( 0 < P(A_{ij} = a_{ij} | X_i, U_i) < 1 \), for all \( i, j \), and \( a_i = (a_{i1}, \ldots, a_{in_i}) \), with \( a_{ij} \in \Omega_A \). When the treatment is binary, all elements in \( a_i \) are binary, and \( a_i \neq 0 \) or \( 1 \).

The unit-level Positivity assumption for binary treatment is \( 0 < P(A_{ij} = 0 | X_i, U_i) < 1 \) and \( 0 < P(A_{ij} = 1 | X_i, U_i) < 1 \). It is not equivalent to Assumption 3 because of the
constraint $a_i \neq 0$ or $1$. Such constraint excludes those clusters that all units in one cluster only received treatment (or control). Besides, the equivalence $P(A_i = a_i|X_i, U_i) = \prod_{j=1}^{n_i} P(A_{ij} = a_{ij}|X_i, U_i)$ is obtained from the second component in Assumption 4.

**Assumption 4 (Cluster-level Ignorability).** $\{Y_i(0), Y_i(1)\} \perp \perp A_i|X_i, U_i$ for all $i$.

Assumption 4 indicates that, in each cluster, all units’ treatment assignments are not affected by the units’ potential outcomes given information of $X_i$ and $U_i$. It is different from the another form of Ignorability assumption, $\{Y_i(0), Y_i(1)\} \perp \perp A_i|X_i$, which indicates no unmeasured confounder. For clustered data, cluster-level confounding factors may be various across clusters. Their existence should not be ignored. Instead, Assumption 4 allows the existence of unmeasured cluster-level confounding variable. A more general form of Assumption 4 is $\{Y_i(a)\}_{a \in \Omega_A} \perp \perp A_i|X_i, U_i$ for all $i$.

Under the aforementioned assumptions, for binary treatment, the IPW estimator for the average treatment effect is expressed as

$$
\tau_{IPW} = \frac{1}{n} \sum_i \sum_j \left\{ \frac{A_{ij}Y_{ij}}{P(A_{ij} = 1|X_i, U_i)} - \frac{(1 - A_{ij})Y_{ij}}{1 - P(A_{ij} = 1|X_i, U_i)} \right\},
$$

where the propensity score $P(A_{ij} = 1|X_i, U_i)$ is the conditional probability of being applied the treatment given $(X_i, U_i)$. In applications, model for unit-level treatment indicator $A_{ij}$ can be constructed using a generalized linear mixed effect model

$$
P(A_{ij} = a|X_i, U_i) = g(X_{ij}^T \beta + U_i),
$$

for all $a$, where $g$ is the link function, and $\beta$ is a $p$-dimensional vector of parameter. For binary treatment indicator, researchers usually choose logic link as the link function. Then we have the following form of a logistic model,

$$
P(A_{ij} = 1|X_{ij}, U_i) = \frac{\exp(X_{ij}^T \beta + U_i)}{1 + \exp(X_{ij}^T \beta + U_i)}.
$$
For multiple treatments, denote by $k$ the treatment level with range $k = 0, \ldots, K$, where $K \leq 1$. Therefore, there are $K + 1$ treatment levels in total. Assume treatment assignment follows a multinomial logistic model. That is,

$$P(A_{ij} = a | \mathbf{X}_{ij}, \mathbf{U}_i) = \frac{\exp\{\sum_{k=1}^{K} I(a = k)(\mathbf{X}_{ij}^T \mathbf{\beta}_k + U_{ik})\} + I(a = 0)}{1 + \sum_{h=1}^{K} \exp(\mathbf{X}_{ij}^T \mathbf{\beta}_h + U_{ih})},$$

where $a = 0, \ldots, K$, $\mathbf{\beta}_k$ is the parameter for $k$th treatment assignment and $U_{ik}$ is the unmeasured cluster-specific variable for the $k$th treatment. Then we have the cluster-specific confounding variable as $\mathbf{U}_i = (U_{i1}, \ldots, U_{iK})$ for the $i$th cluster, with dimension $K$.

We should note that propensity score formulas above involve with the knowledge of $\{\mathbf{U}_i\}_{i=1}^m$, which is unobserved in data. Besides, the existence of unmeasured $\{\mathbf{U}_i\}_{i=1}^m$ is nonignorable. When $\{\mathbf{U}_i\}_{i=1}^m$ are treated as fixed effects and estimated by maximizing the overall likelihood, the estimates tends to be biased as the number of cluster $m$ increases (Neyman and Scott, 1948). Moreover, when $\{\mathbf{U}_i\}_{i=1}^m$ are treated as random effects, the requirement of independence between $\mathbf{U}_i$ and $\mathbf{X}_i$ cannot always be guaranteed. So we are motivated to seek an estimation procedure without directly dealing with $\{\mathbf{U}_i\}_{i=1}^m$. Besides, we want to specify under what conditions, the method is feasible.

### 2.3 Two Theorems Utilizing Sufficient Statistics

Before introducing the proposed method, we introduce two theorems utilizing sufficient statistics. These two theorems provide theoretical foundations to our proposed method. In particular, the new method is constructed by utilizing a sufficient statistic in each cluster.

**Theorem 1.** Suppose $\mathbf{Z}$ and $\mathbf{X}$ are random variables with domain $\Omega_\mathbf{Z}$ and $\Omega_\mathbf{X}$, and $\mathbf{\theta}$ is a parameter vector with domain $\Omega_\mathbf{\theta}$. Let $\mathbf{Z}_{\text{sub}} = (Z_{J_1}, \ldots, Z_{J_k})$ with domain $\Omega_{\mathbf{Z}_{\text{sub}}}$ be a subvector of $\mathbf{Z} = (Z_1, \ldots, Z_n)$, where $\{J_1, \ldots, J_k\} \subseteq \{1, \ldots, n\}$. Let $\mathbf{T}$ be a function of $\mathbf{Z}$ with domain $\Omega_{\mathbf{T}}$ satisfies that for each element $\mathbf{t} \in \Omega_{\mathbf{T}}$, there exist at least two elements, $\mathbf{z}^0, \mathbf{z}^* \in \Omega_{\mathbf{Z}}$ and their corresponding subvectors $\mathbf{z}^0_{\text{sub}}, \mathbf{z}^*_{\text{sub}} \in \Omega_{\mathbf{Z}_{\text{sub}}}$ such that (i) $\mathbf{z}^0_{\text{sub}} \neq \mathbf{z}^*_{\text{sub}}$
and (ii) $T(z^o) = T(z^*) = t$. If $T$ is sufficient for $\theta$, and $0 < P(Z = z | X, \theta) \leq P(Z_{sub} = z_{sub} | X, \theta) < 1$ for any $z \in \Omega_Z$ and its corresponding subvector $z_{sub} \in \Omega_{Z_{sub}}$, then $0 < P\{Z_{sub} = z_{sub} | X, T = T(z)\} < 1$.

The proof of Theorem 1 is in the Supplementary Materials.

Remark 1. Theorem 1 indicates that by utilizing a sufficient statistic $T$ for $\theta$, one can still obtain a non-zero conditional probability of $Z_{sub}$, which does not depend on $\theta$ anymore. It is helpful, when one wants to avoid the involvement of nuisance parameter $\theta$ and maintains the same probability range. Moreover, one should notice that the two probabilities, $P(Z_{sub} = z_{sub} | X, \theta)$ and $P\{Z_{sub} = z_{sub} | X, T = T(z)\}$, are not necessarily the same. Besides, the dimensions of $T$ and $\theta$ are the same (Cox, 2006, Section 2.5). One special case of the theorem is setting $Z_{sub} = Z$. That means we are considering the range of the conditional probability of $Z$, which is $0 < P\{Z = z | X, T = T(z)\} < 1$.

When applying Theorem 1, we have to pay attention to the requirement for the sufficient statistic $T$, which is stronger than surjection. If $T$ is a surjective function, it means for any $t \in \Omega_T$ there exists at least one element $z \in \Omega_Z$ and a corresponding subvector $z_{sub} \in \Omega_{T_{sub}}$ such that $T(z) = t$. In this case, the conclusion in Theorem 1 is changed to $0 < P\{Z_{sub} = z_{sub} | X, T = T(z)\} \leq 1$. This means if $T$ is a surjective function, the probability of $Z_{sub}$ conditional on $T$ can be 1, even though the original probability of $Z_{sub}$ conditional on $(X, \theta)$ is in range $(0,1)$. In order to make the conditional probability $P\{Z_{sub} = z_{sub} | X, T = T(z)\}$ not equal to 1, we have to construct $T$ more restrictive than surjective. That is, we require at least “two” elements rather than “one” element $z^o, z^* \in \Omega_Z$ and corresponding subvectors $z^o_{sub}, z^*_{sub} \in \Omega_{Z_{sub}}$ such that $z^o_{sub} \neq z^*_{sub}$ and $T(z^o) = T(z^*) = t$ for any $t \in \Omega_T$.

**Theorem 2.** Suppose $Z_1, Z_2$ and $Z_3$ are random variables, and the corresponding domains are $\Omega_{Z_1}, \Omega_{Z_2}$ and $\Omega_{Z_3}$, respectively. Let $\theta$ be a parameter with domain $\Omega_\theta$. Let $T$, a function of $Z_1$, be sufficient for $\theta$. If $Z_1 \perp \perp Z_2 | Z_3, \theta$, then $Z_1 \perp \perp Z_2 | Z_3, T$.

The proof of Theorem 2 is in the Supplementary Materials.
Remark 2. Theorem 2 has great potential in applications when dealing with nuisance parameters, which are nonignorable and not of main interest in estimation. Specifically, when two random variables are independent conditional on a nuisance parameter, one can check whether there exist a sufficient statistic, which is a function of the random variable $Z_1$. If such sufficient statistic exists, then a new independence holds, which is conditional on the sufficient statistic rather than the parameter. The new independence is usually more desirable since it only involves with $(Z_1, Z_2, Z_3, T)$, which are usually formed from data. To obtain the independence conditional on the sufficient statistic via Theorem 2, we do not need information on (i) the further requirement of sufficient statistic described in Theorem 1, or (ii) the prior distribution of the parameter $\theta$, or (iii) the relationship between $Z_2$ and $\theta$, or (iv) the relationship between $Z_3$ and $\theta$. It means this theorem has a great property of sufficient statistics in applications. To apply the theorem, one should note that the probability distribution of $Z_1$ conditioned on the parameter $\theta$ should not be misspecified. Besides, the same as discussed in Theorem 1, the dimension of sufficient statistic $T$ should be the same as $\theta$, which was indicated by Cox (2006, Section 2.5).

2.4 Assumptions Conditional on Sufficient Statistics

Sufficient statistics play an important role in the aforementioned two theorems. To utilize them in our proposed method, we simply treat $\{U_i\}_{i=1}^m$ as cluster-specific parameters in Model (2), then we consider the following assumption for sufficient statistics existence.

Assumption 5. For each cluster, there exists a function of $A_i$, defined as $T_i = T_i(A_i)$, is sufficient for $U_i$ in (2). Moreover, for any value $t$ of $T_i$ and any unit $j$, there exist at least two different possible values of $A_{ij}$ in $A_i$, i.e. $a_{ij}$ in $a_i$ and $a_{ij}^*$ in $a_i^*$, such that (i) $a_{ij} \neq a_{ij}^*$ and (ii) $T_i(a_i) = T_i(a_i^*) = t$.

Recall two aforementioned assumptions in Section 2.2, Cluster-level Positivity (Assumption 3) and Cluster-level Ignorability (Assumption 4). Both of them require the information
of cluster-specific confounding variable $U_i$ in each cluster, which is not observed in data. Assume Assumption 5 holds, by Theorems 1 and 2 Assumptions 3 and 4 can be replaced:

**Assumption 3**. The treatment assignment probability conditional on sufficient statistic satisfies $0 < P\{A_{ij} = a_{ij}|X_i, T_i = T(a_i)\} < 1$, for all $i$, $j$, and $a_i = (a_{i1}, \ldots, a_{in_i})$. When the treatment is binary, $a_i \neq 0$ or 1.

**Assumption 4**. ${Y_i(0), Y_i(1)} \perp \perp A_i|X_i, T_i$ for all $i$.

The general form of Assumption 4 is ${Y_i(a)}_{a \in \Omega_A} \perp \perp A_i|X_i, T_i$ for all $i$. The above two assumptions are more preferable to the original Cluster-level Positivity and Cluster-level Ignorability in Assumptions 3 and 4. This is because, by utilizing the sufficient statistics $\{T_i\}_{i=1}^m$ in Theorems 1 and 2 the unmeasured cluster-specific confounding variables $\{U_i\}_{i=1}^m$ can be ignored in Assumptions 3 and 4. Then methods proposed under these two assumptions can also be relaxed from considering $U_i$.

### 3 Proposed Methodology

#### 3.1 Inverse Conditional Probability Weighted (ICPW) estimator

Our proposed estimator is constructed from a conditional probability by utilizing the sufficient statistic. In particular, based on model (2), we construct a probability of $A_{ij}$ conditional on $X_i$ and the sufficient statistic $T_i$ described in Assumption 5.

$$P(A_{ij} = a_{ij}|X_i, T_i; \beta) = \frac{\sum_{a^* \in \Omega_{i,j}} P(A_i = a^*_i|X_i, U_i; \beta)}{\sum_{\tilde{a} \in \tilde{\Omega}_i} P(A_i = \tilde{a}_i|X_i, U_i; \beta)}$$

for all $i$, $j$, and any value $a_i$ in the domain $\Omega_i$. The set $\Omega_{i,j}$ in the numerator of (5) is a set of all possible treatments $a^* = (a^*_1, \ldots, a^*_n)$ satisfying two criteria – (i) the $j$th components is the same as the observed value, i.e., $a^*_j = a_{ij}$; (ii) the value of $T_i(a^*)$ equals to the value of $T_i(a_i)$ from data. In short, $\Omega_{i,j} = \{a^* \in \Omega_i|a^*_j = a_{ij} \text{ and } T_i(a^*) = T_i(a_i)\}$. The other set $\tilde{\Omega}_i$ in the denominator of (5) is defined as $\tilde{\Omega}_i = \{\tilde{a} \in \Omega_i|T_i(\tilde{a}) = T_i(a_i)\}$. In particular,
\( \tilde{\Omega}_i \) contains all possible permutations of treatments within one cluster such that the function \( T_i \) of each permutation is the same as that of the observed treatments in the cluster. For all units in the \( i \)th cluster, we assign each unit a weight defined as the inverse of the conditional probability described in (5). The conditional probability is an important component in the proposed method. So the inverse conditional probability weighted (ICPW) estimator for \( E\{Y(a)\} \) is \( Y_{ICPW}(a) = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{I(A_{ij} = a)}{P(A_{ij} = a | X_i, T_i; \beta)} Y_{ij} \).

Instead of unit-level unbiasedness, we show that our proposed weighting method is cluster-level unbiased. That is, suppose \( E\{Y_{ij}(a)\} \) is finite for all \( i, j \) and \( a \), then for a cluster-level potential outcome sum \( \sum_{j=1}^{n_i} E\{Y_{ij}(a)\} \) with treatment level \( a \),

\[
E\left\{ \sum_{j=1}^{n_i} \frac{I(A_{ij} = a)}{P(A_{ij} = a | X_i, T_i; \beta)} Y_{ij} \right\} = \sum_{j=1}^{n_i} E_{X_i, T_i} \left\{ E_{A_{ij}, Y_{ij}(a) | X_i, T_i} \left\{ \frac{I(A_{ij} = a)}{P(A_{ij} = a | X_i, T_i; \beta)} Y_{ij}(a) \right\} | X_i, T_i \right\} \\
= \sum_{j=1}^{n_i} E_{X_i, T_i} \left\{ E_{A_{ij} | X_i, T_i} \left\{ \frac{I(A_{ij} = a)}{P(A_{ij} = a | X_i, T_i; \beta)} | X_i, T_i \right\} E_{Y_{ij}(a) | X_i, T_i} \left\{ Y_{ij}(a) | X_i, T_i \right\} \right\} \\
= \sum_{j=1}^{n_i} E_{Y_{ij}(a)} \left\{ Y_{ij}(a) \right\}. \tag{6}
\]

The above equation holds due to Assumptions [2] [4] and \( E\left\{ \frac{I(A_{ij} = a)}{P(A_{ij} = a | X_i, T_i; \beta)} | X_i, T_i \right\} = 1 \).

Therefore, for binary treatment, the corresponding ICPW estimator of the average treatment causal effect based on conditional probability described in (5) is

\[
\tau_{ICPW} = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \left\{ \frac{A_{ij} Y_{ij}}{P(A_{ij} = 1 | X_i, T_i; \beta)} - \frac{(1 - A_{ij}) Y_{ij}}{1 - P(A_{ij} = 1 | X_i, T_i; \beta)} \right\}. \tag{7}
\]

For the aforementioned logistic model (3), the sufficient statistic is the treatment sum in the cluster \( T_i = \sum_{j=1}^{n_i} A_{ij} \). Then the probability conditional on sufficient statistic is

\[
P(A_{ij} = a_{ij} | X_i, \sum_{j=1}^{n_i} A_{ij} = \sum_{j=1}^{n_i} a_{ij}; \beta) = \frac{\exp(a_{ij} X_{ij}^T \beta) \sum_{a^* \in \hat{V}_{ij}} \exp(\sum_{m=1,m \neq j}^{n_i} a^*_i X_{ij}^T \beta)}{\sum_{a \in \hat{V}_i} \exp(\sum_{m=1}^{n_i} a_i X_{ij}^T \beta)}. \tag{8}
\]
for all $i, j$, and $a_{ij} = 0, 1$. Set $\mathbb{V}_{i,j}$ in the numerator of (8) is a set of all possible treatments $\mathbf{a}^* = (a^*_1, \ldots, a^*_n)$ satisfying two criteria – (i) the $j^\text{th}$ components is the same as the observed value, i.e. $a^*_j = a_{ij}$; (ii) the component sum in $\mathbf{a}^*$ equals to the unit treatment sum in one cluster in the dataset, i.e. $\sum_{l=1}^n a^*_l = \sum_{l=1}^n a_{il}$. The definition notation of $\mathbb{V}_{i,j}$ is $\mathbb{V}_{i,j} = \{\mathbf{a}^* \in \{0, 1\}^n | a^*_j = a_{ij} \text{ and } \sum_{l=1}^n a^*_l = \sum_{l=1}^n a_{il}\}$. The other set $\tilde{\mathbb{V}}_i$ in the denominator of (8) has components $\tilde{\mathbf{a}} = (\tilde{a}_1, \ldots, \tilde{a}_n)$. The set is defined as $\tilde{\mathbb{V}}_i = \{\tilde{\mathbf{a}} \in \{0, 1\}^n | \sum_{l=1}^n \tilde{a}_l = \sum_{l=1}^n a_{il}\}$.

Specifically, $\tilde{\mathbb{V}}_i$ contains all possible permutations of treatments within a cluster such that the each permutation sum equals to the observed treatment sum in the cluster from data.

Moreover, because $\mathbf{a}_i \neq \mathbf{0}, \mathbf{1}$, the conditional probabilities, $P\{\mathbf{A}_i = \mathbf{0}| \mathbf{X}_i, \sum_{j=1}^n A_{ij} = 0\} = P\{\mathbf{A}_i = 1| \mathbf{X}_i, \sum_{j=1}^n A_{ij} = n_i\} = 1$, are excluded in Assumption 4. It is similar to the method proposed by Skinner and D’arrigo (2011), where the nonresponse indicator is treated as a binary treatment. The above ICPW method can be summarized in Algorithm 1

**Algorithm 1** Inverse conditional probability weighted (ICPW) estimator for $\tau$ with binary treatment

0: Let the treatment $A_{ij}$ for unit $j$ in cluster $i$ follows model (3), there exists a function of $\mathbf{A}_i$, defined $T_i$, satisfies Assumption 5
1: Obtain the conditional maximum likelihood estimator $\hat{\beta}$ by maximizing the joint conditional likelihood (11) with $a_{ij} = 0$ or 1.
2: Compute the conditional probability with the conditional maximum likelihood estimator $\hat{\beta}$. That is, compute $P(A_{ij} = 1| \mathbf{X}_i, T_i; \hat{\beta})$.
3: Compute the ICPW estimator $\hat{\tau}_{ICPW}$ for $\tau$

$$
\hat{\tau}_{ICPW} = \frac{1}{n} \sum_{i=1}^m \sum_{j=1}^{n_i} \left\{ \frac{A_{ij}Y_{ij}}{P(A_{ij} = 1| \mathbf{X}_i, T_i; \hat{\beta})} - \frac{(1 - A_{ij})Y_{ij}}{1 - P(A_{ij} = 1| \mathbf{X}_i, T_i; \hat{\beta})} \right\}, \quad (9)
$$

For multiple treatments, e.g. the aforementioned multinomial logistic model (4), the sufficient statistic for $\mathbf{U}_i$ is $\mathbf{T}_i = (T_{i0}, \ldots, T_{i(K-1)})$, where $T_{ik} = \sum_{j=1}^{n_i} I(A_{ij} = k)$ for $k = 0, \ldots, K - 1$. Then the conditional probability for $A_{ij}$ conditional on $\mathbf{T}_i$ is

$$
P(A_{ij} = a_{ij}| \mathbf{X}_{ij}, \mathbf{T}_i) = \frac{\exp\{\sum_{k=1}^K I(a_{ij} = k) \mathbf{X}_{ij}^T \beta_k + I(a_{ij} = 0)\} \lambda_{ij}}{\sum_{\tilde{\mathbf{a}} \in \tilde{\mathbb{V}}_i} \exp\left[\sum_{l=1}^{n_i} (\sum_{k=1}^K I(\tilde{a}_l = k) \mathbf{X}_{ij}^T \beta_k + I(\tilde{a}_l = 0)\right]}, \quad (10)
$$
where \( \lambda_{ij} = \sum_{a^* \in V_{i,j}} \exp \left[ \sum_{l=1}^{n_i} \sum_{k=1}^{K} I(a^*_l = k) X^T_{ij} \beta_k + I(a^* = 0) \right] \). Set \( V_{i,j} \) in the above equation is a set of all possible treatments \( a^* = (a^*_1, \ldots, a^*_n) \) satisfying two criteria – (i) the \( j \)th components is the same as the observed value, i.e. \( a^*_j = a_{ij} \); (ii) the component sum in \( a^* \) equals to the unit treatment sum in one cluster in the dataset for each treatment category in each cluster \( T_i(a^*_i) = T_i(a_i) \), i.e. \( \sum_{l=1}^{n_i} I(a^*_l = k) = \sum_{l=1}^{n_i} I(a_l = k) \) for \( k = 1, \ldots, K \).

The definition notation of \( V_{i,j} \) is \( V_{i,j} = \{ a^* \in \{0,1,\ldots,K\}^n | a^*_j = a_{ij} \text{ and } \sum_{l=1}^{n_i} I(a^*_l = k) = \sum_{l=1}^{n_i} I(a_l = k) \text{ for all } k \} \). The set \( \tilde{V}_i \) has components \( \tilde{a} = (\tilde{a}_1, \ldots, \tilde{a}_n) \). It is defined as \( \tilde{V}_i = \{ \tilde{a} \in \{0,1,\ldots,K\}^n | \sum_{l=1}^{n_i} I(\tilde{a}_l = k) = \sum_{l=1}^{n_i} I(a_l = k) \text{ for all } k \} \). Specifically, \( \tilde{V}_i \) contains all possible permutations of treatment \( k \)th category within a cluster such that the each permutation sum equals to the observed treatment sum in the cluster from data. The algorithm for our proposed method is summarized in Algorithm 2.

**Algorithm 2** Inverse conditional probability weighted (ICPW) estimator for \( E\{Y(a)\} \)

0: Let the treatment \( A_{ij} \) for unit \( j \) in cluster \( i \) follows model (2), there exists a function of \( A_i \), defined \( T_i \), satisfies Assumption 5.

1: Obtain the conditional maximum likelihood estimator \( \hat{\beta} \) by maximizing the joint conditional likelihood

\[
L^c(\beta) = \prod_{i=1}^{m} \prod_{j=1}^{n_i} P(A_{ij} = a_{ij} | X_i, T_i; \beta),
\]

where \( P(A_{ij} = a_{ij} | X_i, T_i; \beta) \) is described in [5].

2: Compute the conditional probability with the conditional maximum likelihood estimator \( \hat{\beta} \). That is, compute \( P(A_{ij} = a | X_i, T_i; \beta) \).

3: Compute the ICPW estimator \( \hat{Y}_{ICPW}(a) \) for \( E\{Y(a)\} \)

\[
\hat{Y}_{ICPW}(a) = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{I\{A_{ij} = a\} Y_{ij}}{P(A_{ij} = a | X_i, T_i; \beta)}.
\]

### 3.2 Robustness of ICPW estimator

An attractive property of the ICPW estimator is its robustness, which is summarized below.

**Theorem 3.** The proposed ICPW estimator is robust to both (i) the correlation between the unmeasured cluster-specific confounding variable and the covariates and (ii) the correlation...
between the unmeasured cluster-specific confounding variable and the outcome.

The proof is in the Supplementary Materials.

Remark 3. Theorem 3 illustrates the unbiasedness holds no matter the correlation between $X_i$ and $U_i$ (i.e., $\rho_{X,U}$ in Figure 2), or the correlation between $Y_i$ and $U_i$ (i.e., $\rho_{Y,U}$ in Figure 2), or the characteristics of $U_i$ (e.g. its distribution) is. Since $U_i$s are not observed in the real data, the two correlations $\rho_{X,U}$ and $\rho_{Y,U}$ in Figure 2 are usually unobserved too. Such robust property exhibits an advantage of ICPW method in that it comes with more flexibility and confidence in estimating the average causal effect.

![Figure 2: A graph representing two possible correlations with respect to the unmeasured cluster-specific confounding variable $U_i$. One is the correlation ($\rho_{X,U}$) between the covariates $X_i$ and $U_i$. The other one is the correlation ($\rho_{Y,U}$) between the outcome $Y_{ij}$ and $U_i$.](image)

4 Asymptotic Properties

The main goal of this section is to show the asymptotic properties of the ICPW estimator as $n \to \infty$. To reach this goal, we first focus on the asymptotic properties of conditional maximum likelihood estimator (CMLE) of $\beta$. To make sure the CMLE of $\beta$ is uniquely determined, we consider the minimal sufficient statistic $T_i$ for $U_i$ for all $i$. This is because, as stated in Andersen (1970), the conditional probability has less information about $U_i$ if $T_i$ is not minimum sufficient. Next, we prove the asymptotic properties of the ICPW estimator of $E[Y(a)]$ for all treatment level $a \in \Omega_A$. Lastly, the asymptotic properties of the ICPW estimator of $\tau$ with binary treatment can be proved by Delta method using Taylor series
expansion. Here we consider the asymptotic results with respect to the number of clusters. That is, we will investigate the asymptotic properties of ICPW estimator with respect to \( m \) when \( n_i \)'s are fixed and bounded.

### 4.1 Asymptotic Properties of CMLE for \( \beta \)

Andersen (1970) proved that the conditional maximum likelihood estimates are consistent and asymptotically normally distributed under regularity conditions. We adopt Andersen (1970)'s results to show the asymptotic properties of CMLE for \( \beta \).

**Theorem 4.** (Consistency of CMLE for \( \beta \)) Suppose that Assumption 1 and the Conditions 1-3 specified in the Web Appendix A hold, and the treatment assignment follows the cluster-specific model in (2), and there exist sufficient statistics \( T_i \) as specified in Assumption 5 and \( \max_{1 \leq i \leq m} n_i / n \to 0 \) as \( n \to \infty \). The CMLE \( \hat{\beta} \) can be obtained by maximizing the joint conditional likelihood \( \prod_{i=1}^{m} \prod_{j=1}^{n_i} P(A_{ij} = a_{ij} | X_i, T_i; \beta) \), where \( P(A_{ij} = a_{ij} | X_i, T_i; \beta) \) is specified in (5). Therefore, \( \hat{\beta} \) is a consistent estimate for \( \beta \).

**Theorem 5.** (Asymptotic Normality of CMLE for \( \beta \)) Suppose that Assumption 1 and the Conditions 1-5 specified in the Web Appendix A hold, and the treatment assignment follows the cluster-specific model in (2), and there exist sufficient statistics \( T_i \) for all \( i \) as specified in Assumption 5 and \( \max_{1 \leq i \leq m} n_i / n \to 0 \) as \( n \to \infty \). The CMLE \( \hat{\beta} \) can be obtained by maximizing the joint conditional likelihood \( \prod_{i=1}^{m} \prod_{j=1}^{n_i} P(A_{ij} = a_{ij} | X_i, T_i; \beta) \), where \( P(A_{ij} = a_{ij} | X_i, T_i; \beta) \) is specified in (5). Let \( \phi_{ij}(a | X_i, T_i; \beta) \) represent the conditional probability density function for \( P(A_{ij} = a | X_i, T_i; \beta) \), which is continuous and differentiable with respect to \( \beta \) at \( \beta_0 \). Then we have \( \sqrt{n} (\hat{\beta} - \beta_0) \to N(0, B_1(\beta_0)) \) in distribution as \( n \to \infty \), where \( B_1(\beta) = \{B_2(\beta)\}^{-1} B_3(\beta) \{B_2(\beta)\}^{-1} \) with

\[
B_2(\beta) = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} E \left\{ \frac{\partial}{\partial \beta} \frac{\partial}{\partial \beta^T} \log \phi_{ij}(A_{ij} | X_i, T_i, \beta) \right\}
\]
and

\[ B_3(\beta) = \frac{1}{n} \sum_{i=1}^{m} E \left[ \left\{ \frac{\partial}{\partial \beta} \log \phi_{ij}(A_{ij}|X_i, T_i; \beta) \right\} \left\{ \frac{\partial}{\partial \beta^T} \log \phi_{ij}(A_{ij}|X_i, T_i; \beta) \right\} \right]. \]

The proofs of Theorems 4 and 5 are skipped here since they are conceptually the same as Andersen (1970)’s proof. The difference is that Andersen’s work was not aimed to clustered data structure. To adopt his work to clustered data, we treat the cluster-level joint probability \( \prod_{j=1}^{n_i} \phi_{ij}(A_{ij}|X_i, T_i; \beta) \) as the unit probability in his proof. Therefore, the consistency result can be obtained with respect to the number of clusters (i.e. \( n \to \infty \) as \( m \to \infty \)).

4.2 Asymptotic Properties of ICPW estimator for \( E[Y(a)] \)

In Section 3.1, we have shown in (6) that the proposed ICPW estimator is an unbiased estimator for one cluster. Therefore, it is straightforward to show the overall unbiasedness:

\[
\begin{align*}
E \left\{ \frac{1}{n} \sum_{i} \sum_{j} \frac{1}{P(A_{ij}=a)} \frac{I\{A_{ij}=a\} Y_{ij}}{\phi_{ij}(A_{ij}|X_i, T_i; \beta)} \right\} &= \frac{1}{n} \sum_{i} \sum_{j} E \{ Y_{ij}(a) \} = E \{ Y(a) \}, \forall a \in \Omega_A. \tag{13}
\end{align*}
\]

The corresponding estimator for \( E\{Y(a)\} \), i.e., \( \hat{Y}_{ICPW}(a) \), is defined in (12) in Algorithm 2. The asymptotic properties of \( \hat{Y}_n(a) \) is shown in Theorem 6.

**Theorem 6. (Asymptotic Normality of ICPW estimator for \( E[Y(a)] \))** Suppose \( \sqrt{n}(\hat{\beta} - \beta_0) \to N(0, B_1(\beta_0)) \) and \( \phi_{ij}(a|X_i, T_i; \beta) \) is continuous and differentiable with respect to \( \beta \) at \( \beta_0 \), with \( \frac{\partial \phi_{ij}(a|X_i, T_i; \beta)}{\partial \beta} \bigg|_{\beta=\beta_0} \neq 0 \). Let \( \hat{Y}_n(\beta_0; a) \) be the ICPW estimator at \( \beta = \beta_0 \), and \( E\{Y(a)\} \) be the expectation of the ICPW estimator at \( \beta = \beta_0 \). Assume \( \sigma^2_{1,\beta_0} = E\{\hat{Y}_n(\beta_0; a) - E\{Y(a)\}\}^2 \) is bounded. Then the ICPW estimator in (12) satisfies \( \sqrt{n}V_1(\beta_0)^{-1/2}(\hat{Y}_{ICPW}(a) - E\{Y(a)\}) \to N(0, 1) \) in distribution as \( n \to \infty \), where \( V_1(\beta_0) = E\{H_1(\beta_0)^T B_1(\beta_0) H_1(\beta_0)\} \) is assumed to be bounded and positive, and \( H_1(\beta) = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{I\{A_{ij}=a\} Y_{ij}}{\phi_{ij}(a|X_i, T_i; \beta)} \frac{\partial \phi_{ij}(a|X_i, T_i; \beta)}{\partial \beta} \).

The proof is in the Supplementary Materials.
4.3 Asymptotic Properties of ICPW estimator for $\tau$

From the results in (13) in Section 4.2, we know the ICPW estimator for binary treatment is unbiased for $\tau$. The asymptotic properties of $\hat{\tau}_{ICPW}$ defined in (9) is presented below:

Theorem 7. (Asymptotic Normality of ICPW estimator for $\tau$) For binary treatment, let $\phi_{ij}(\beta)$ represent the conditional probability density function of $P(A_{ij} = 1|X_i, T_i; \beta)$. Suppose $\sqrt{n}(\hat{\beta} - \beta_0) \rightarrow N(0, B_1(\beta_0))$ and $\phi_{ij}(\beta)$ is continuous and differentiable with respect to $\beta$ at $\beta_0$, with $\frac{\partial \phi_{ij}(\beta)}{\partial \beta}|_{\beta=\beta_0} \neq 0$. $\tau$ is the true average causal effect at $\beta = \beta_0$, and let $\hat{\tau}_n(\beta_0)$ be the ICPW estimator at $\beta = \beta_0$. Assume $\sigma^2_{\beta_0} = E[\hat{\tau}_n(\beta_0) - \tau]^2$ is bounded. Then the ICPW estimator in (9) satisfies $\sqrt{n}V_2(\beta_0)^{-1/2}(\hat{\tau}_{ICPW} - \tau) \rightarrow N(0, 1)$ in distribution as $n \rightarrow \infty$, where $V_2(\beta_0) = E\{H_2(\beta_0)^TB_1(\beta_0)H_2(\beta_0)\}$, which is assumed to be bounded and positive, and $H_2(\beta) = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \left\{ \frac{A_{ij}}{\phi_{ij}(\beta)} + \frac{1-A_{ij}}{[1-\phi_{ij}(\beta)]^2} \right\} Y_{ij} \frac{\partial \phi_{ij}(\beta)}{\partial \beta}$.

The proof is in the Supplementary Materials.

5 Simulation Studies

We conduct two simulation studies to show the robustness of the ICPW estimator. In the first simulation study, we specify the number of clusters to be $m = 500$, and the cluster size ($n_i$) to be the integer part of $D_i \sim Unif(2, 6)$. So cluster sizes range from 2 to 5. In comparison, the second simulation study has smaller data size. There are with 20 clusters ($m = 20$) and the cluster size is the integer part of $D_i \sim Unif(2, 21)$, indicating a range from 2 to 20. Two covariates, a continuous covariate $X_{1,ij}$ and a categorical covariate $X_{2,ij}$, are generated independently for each unit. In particular, $X_{1,ij} \sim N(0, 1)$ and $X_{2,ij} = -1$, or 0, or 1 with equal probabilities. The cluster-specific confounding variable $U_i \sim N(-\rho X_{iU}[\bar{X}_{1,i} + \bar{X}_{2,i}], 1)$, where $\bar{X}_{1,i}$ and $\bar{X}_{2,i}$ are the means over the units within one cluster. We change the value of $\rho_{X,U}$ to manipulate the correlation between the covariates and $U_i$’s. Note that the expectation of $U_i$ is always 0 for any $\rho_{X,U}$. The treatment assignment mechanism is $P(A_{ij} = 1|X_i, U_i) = \exp(X_{1,ij} + X_{2,ij} + U_i)/(1 + \exp(X_{1,ij} + X_{2,ij} + U_i))$. For each unit, two potential
outcomes are generated as $Y_{ij}(0) = X_{1,ij} + X_{2,ij} + e_{ij}^0$ and $Y_{ij}(1) = X_{1,ij} + X_{2,ij} + \tau + \rho_{Y,U} U_i + e_{ij}^1$, where $\tau = 2$, $e_{ij}^0, e_{ij}^1 \sim N(0,1)$, and $\rho_{Y,U}$ controls the correlation between the causal effect and $U_i$’s. The observed outcomes follow Assumption 2. We consider four scenarios:

1. $(\rho_{X,U}, \rho_{Y,U})=(0,0)$. The cluster-specific confounding variable $U_i$ is independent of both the covariate $X_i$ and the causal effect $Y_{ij}(1) - Y_{ij}(0)$;

2. $(\rho_{X,U}, \rho_{Y,U})=(5,0)$. The cluster-specific confounding variable $U_i$ is correlated of the covariate $X_i$, and it is independent with the causal effect $Y_{ij}(1) - Y_{ij}(0)$;

3. $(\rho_{X,U}, \rho_{Y,U})=(0,5)$. The cluster-specific confounding variable $U_i$ is correlated of the causal effect $Y_{ij}(1) - Y_{ij}(0)$, and it is independent with the covariate $X_i$;

4. $(\rho_{X,U}, \rho_{Y,U})=(5,5)$. The cluster-specific confounding variable $U_i$ is correlated with both the covariate $X_i$ and the causal effect $Y_{ij}(1) - Y_{ij}(0)$.

We obtain an estimator from each simulated data, i.e., $\hat{\tau}_{simu} = 1/n \sum_{i=1}^{n} \sum_{j=1}^{m} \{Y_{ij}(1) - Y_{ij}(0)\}$. Note that $\hat{\tau}_{simu}$ can not be obtained from the real data due to fundamental problem in causal inference (Rubin, 1974; Holland, 1986). Therefore $\hat{\tau}_{simu}$ and $se(\hat{\tau}_{simu})$ are not used for comparison to other methods, but for an illustration of the true causal effect and its corresponding standard error obtained from simulated data.

For method comparison, we consider four estimators for $\tau$. The first is $\hat{\tau}_{naive}$, which is a simple estimator without weight adjustment, i.e., $\hat{\tau}_{naive} = 1/n \{A_{ij} Y_{ij} - (1 - A_{ij}) Y_{ij}\}$. The second estimator $\hat{\tau}_{IPW,ran}$ is an IPW estimator in (1) by specifying (2) as a logistic mixed effects model where cluster-specific effect is random. The third estimator $\hat{\tau}_{IPW,fix}$ is an IPW estimator in (1) by specifying (2) as a logistic model where cluster-specific effect is fixed effect. The last estimator $\hat{\tau}_{ICPW}$ is the proposed estimator obtained from Algorithm 1.

Simulation results are presented in Tables 1 and 2. Each simulation study is conducted in R and are repeated 1,000 times. The simple estimator $\hat{\tau}_{naive}$ shows large bias in general. The IPW estimator $\hat{\tau}_{IPW,ran}$ is biased when $U_i$ is correlated with either covariates or
Method Estimate Bias to $\tau$ s.e. | Method Estimate Bias to $\tau$ s.e.
---|---
### Scenario 1: ($\rho_{X,U}, \rho_{Y,U})=(0,0)$
$\hat{\tau}_{\text{simu}}$ | 2.000 | 0.000 | 0.034 | $\hat{\tau}_{\text{simu}}$ | 2.000 | 0.000 | 0.034
$\hat{\tau}_{\text{naive}}$ | 1.594 | -0.406 | 0.040 | $\hat{\tau}_{\text{naive}}$ | 1.370 | -0.630 | 0.042
$\hat{\tau}_{\text{IPW,ran}}$ | 2.009 | 0.009 | 0.072 | $\hat{\tau}_{\text{IPW,ran}}$ | 1.914 | -0.086 | 0.063
$\hat{\tau}_{\text{IPW,fix}}$ | 1.843 | -0.157 | 0.562 | $\hat{\tau}_{\text{IPW,fix}}$ | 1.906 | -0.094 | 0.522
$\hat{\tau}_{\text{ICP W}}$ | 2.003 | 0.003 | 0.148 | $\hat{\tau}_{\text{ICP W}}$ | 2.003 | 0.003 | 0.137
### Scenario 3: ($\rho_{X,U}, \rho_{Y,U})=(0,5)$
$\hat{\tau}_{\text{simu}}$ | 2.000 | 0.000 | 0.236 | $\hat{\tau}_{\text{simu}}$ | 2.052 | 0.052 | 0.664
$\hat{\tau}_{\text{naive}}$ | 2.022 | 0.222 | 0.140 | $\hat{\tau}_{\text{naive}}$ | 3.414 | 1.414 | 0.361
$\hat{\tau}_{\text{IPW,ran}}$ | 3.100 | 1.100 | 0.285 | $\hat{\tau}_{\text{IPW,ran}}$ | 8.746 | 6.746 | 0.669
$\hat{\tau}_{\text{IPW,fix}}$ | 1.619 | -0.381 | 1.505 | $\hat{\tau}_{\text{IPW,fix}}$ | 0.996 | -1.004 | 2.913
$\hat{\tau}_{\text{ICP W}}$ | 2.005 | 0.005 | 0.400 | $\hat{\tau}_{\text{ICP W}}$ | 2.089 | 0.089 | 1.016
### Scenario 4: ($\rho_{X,U}, \rho_{Y,U})=(5,5)$
$\hat{\tau}_{\text{simu}}$ | 2.000 | 0.000 | 0.236 | $\hat{\tau}_{\text{simu}}$ | 2.052 | 0.052 | 0.664
$\hat{\tau}_{\text{naive}}$ | 2.022 | 0.222 | 0.140 | $\hat{\tau}_{\text{naive}}$ | 3.414 | 1.414 | 0.361
$\hat{\tau}_{\text{IPW,ran}}$ | 3.100 | 1.100 | 0.285 | $\hat{\tau}_{\text{IPW,ran}}$ | 8.746 | 6.746 | 0.669
$\hat{\tau}_{\text{IPW,fix}}$ | 1.619 | -0.381 | 1.505 | $\hat{\tau}_{\text{IPW,fix}}$ | 0.996 | -1.004 | 2.913
$\hat{\tau}_{\text{ICP W}}$ | 2.005 | 0.005 | 0.400 | $\hat{\tau}_{\text{ICP W}}$ | 2.089 | 0.089 | 1.016

Table 1: Results of simulation study 1 based on 1,000 repetitions. Each repetition contains 500 clusters and the cluster size range from 2 to 5. The expected average causal effect is $\tau = 2$. For each method, the estimate, bias to the expected average causal effect, and standard error (s.e.) are reported.

the causal effect. Its bias becomes the largest in scenario 4. In comparison, the bias of $\hat{\tau}_{\text{IPW,fix}}$ is not that large in both simulation studies. But $\hat{\tau}_{\text{IPW,fix}}$ has the largest variance across all scenarios. This is resulted from the Neyman-Scott incidental parameter problem (Neyman and Scott, 1948). In particular, the variance of $\hat{\tau}_{\text{IPW,fix}}$ is increased by the involvement of the cluster-specific parameters. Our proposed estimator $\hat{\tau}_{\text{ICP W}}$ works well across all scenarios in both simulation studies. This confirms Theorem 3 that the ICPW estimator is robust when cluster-specific confounding variable is correlated with the covariates and/or the causal effect.

### 6 A Case Study

For real data analysis, we apply the ICPW method to the low birth weight data from Hosmer and Lemeshow (2000). The data was collected from 189 women in 1986. Among these women, 59 had low-birth-weight babies and 130 had normal-weight babies. They were grouped according to their age. We are interested in estimating the average causal effect ($\tau$) of mother smoking behavior ($A = 1$ if yes and 0 if no smoking) to the baby birth weight in
Method Estimate Bias to $\tau$ s.e. | Method Estimate Bias to $\tau$ s.e.
--- | --- | --- | --- | --- | ---
Scenario 1: $(\rho_{X,U}, \rho_{Y,U})=(0,0)$ | Scenario 2: $(\rho_{X,U}, \rho_{Y,U})=(5,5)$
$\hat{\tau}_{\text{simu}}$ | 2.001 | 0.001 | 0.097 | $\hat{\tau}_{\text{simu}}$ | 2.006 | 0.006 | 0.097
$\hat{\tau}_{\text{naive}}$ | 1.578 | -0.422 | 0.144 | $\hat{\tau}_{\text{naive}}$ | 1.375 | -0.625 | 0.163
$\hat{\tau}_{\text{IPW,ran}}$ | 1.885 | -0.115 | 0.288 | $\hat{\tau}_{\text{IPW,ran}}$ | 1.405 | -0.595 | 0.485
$\hat{\tau}_{\text{IPW,fix}}$ | 1.981 | -0.019 | 0.438 | $\hat{\tau}_{\text{IPW,fix}}$ | 1.973 | -0.027 | 0.618
$\hat{\tau}_{\text{ICP W}}$ | 2.010 | 0.010 | 0.366 | $\hat{\tau}_{\text{ICP W}}$ | 2.007 | 0.007 | 0.442

Table 2: Results of simulation study 2 based on 1,000 repetitions. Each repetition contains 20 clusters and the cluster size range from 2 to 20. The expected average causal effect is $\tau = 2$. For each method, the estimate, bias to the expected average causal effect, and standard error (s.e.) are reported.

grams ($Y$) among these women. After excluding clusters that violate the Assumption 3, we have 182 women in 20 clusters ($m = 20$). In each cluster, there are 2 to 18 women ($n_i$ ranges from 2 to 18). The covariates include race ($X_1$: white, black, and other), number of false premature labors ($X_2$), and standardized mother’s weight at last menstrual period ($X_3$).

Similar to the simulation studies, four methods are considered here: (i) the simple estimator, $\hat{\tau}_{\text{naive}}$, without any weight adjustment; (ii) $\hat{\tau}_{\text{IPW,ran}}$, the IPW estimator by fitting a logistic mixed effects model to the treatment, where the linear predictors include all three covariates and the cluster-specific effect is random; (iii) $\hat{\tau}_{\text{IPW,fix}}$, the IPW estimator by fitting a logistic model similar to the model in (ii) except the cluster-specific effect is fixed effect; (iv) $\hat{\tau}_{\text{ICP W}}$, the proposed ICPW method, where the linear predictors include all three covariates.

Results with 100 bootstrap replicates are displayed in Table 3. Among all estimates for the average causal effect, three estimates, except $\hat{\tau}_{\text{IPW,ran}}$, are negative. The negative causal effect estimate indicates that mother smoking behavior reduces baby birth weight. The ICPW method presents a negative causal effect estimate. The corresponding 95% confidence interval includes zero, indicating non-significant causal effect among these women.
Moreover, we find some similarities by comparing this case study to the scenario 4 of simulation study 2 in the previous section. First, the number of clusters ($m$) and cluster size range in both real data and simulated data are very close. Second, estimator $\hat{\tau}_{IPW,ran}$ shows great difference to both $\hat{\tau}_{IPW,fix}$ and $\hat{\tau}_{ICPW}$. Third, the standard error of $\hat{\tau}_{IPW,fix}$ is greater than that of $\hat{\tau}_{ICPW}$. From these similarities, our conjecture is that the unmeasured cluster-specific confounding factors may be correlated with the covariates and the causal effect as the setting in scenario 4. This seems reasonable in this study that mother’s age may be correlated with mother’s covariates and baby’s birth weight.

| Method      | Estimate | s.e.  | 95% c.i.          |
|-------------|----------|-------|------------------|
| $\hat{\tau}_{naive}$ | -705.9   | 46.6  | (-797.7, -628.9) |
| $\hat{\tau}_{IPW,ran}$ | 194.2     | 1353.3| (-1445.9, 4787.0) |
| $\hat{\tau}_{IPW,fix}$ | -283.4    | 1898.7| (-2985.0, 2864.2) |
| $\hat{\tau}_{ICPW}$    | -227.6    | 402.3 | (-1108.7, 429.7)  |

Table 3: Results of case study based on 100 bootstrap replicates. For each method, the estimate, standard error (s.e.), and 95% confidence interval (c.i.) are reported.

7 Discussion

The ICPW method is attractive for two reasons. First, it is robust to both correlation between $U_i$ and the covariates, and the correlation between $U_i$ and the outcome. Since $U_i$ is unmeasured in data, it is usually difficult or impossible to obtain its correlations to other measured variables. Such correlations can result biased causal effect estimates in many methods. Comparatively, the robustness of ICPW method can overcome the unclear correlations. Second, we do not have to make any further assumptions on $U_i$. Such assumptions include assuming $U_i$ is a random effect, or is a fixed effect, or follows a prior distribution. The relaxeditness of further assumptions on $U_i$ makes it more adaptable in estimation.

Besides, it should be noted that our focus in this paper is the theoretical study of the ICPW method. In addition to the appealing theoretical properties, there are still some future work on the ICPW method that are worth exploring. First, when the cluster size is
large, the computational load for implementing the ICPW method might increase. In particular, we need to consider all possible permutations in both the numerator and denominator of (5). It will be a topic for future research to design numerical algorithms for computing the ICPW estimator efficiently under large cluster sizes. Second, the proposed ICPW method is originated from the simplest format of the IPW method. There are opportunities to make modifications to the ICPW method under more complex settings, for example time-varying treatment causal effect estimation.

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References

Andersen, E. B. (1970). Asymptotic properties of conditional maximum-likelihood estimators. *Journal of the Royal Statistical Society, Series B*, 283–301.

Arpino, B. and M. Cannas (2016). Propensity score matching with clustered data. An application to the estimation of the impact of caesarean section on the apgar score. *Statistics in Medicine* 35(12), 2074–2091.

Arpino, B. and F. Mealli (2011). The specification of the propensity score in multilevel observational studies. *Computational Statistics & Data Analysis* 55(4), 1770–1780.

Austin, P. C. and E. A. Stuart (2015). Moving towards best practice when using inverse probability of treatment weighting (iptw) using the propensity score to estimate causal treatment effects in observational studies. *Statistics in Medicine* 34(28), 3661–3679.

Brumback, B. A. and Z. He (2011). Adjusting for confounding by neighborhood using complex survey data. *Statistics in Medicine* 30(9), 965–972.
Brumback, B. A., M. A. Hernán, S. J. Haneuse, and J. M. Robins (2004). Sensitivity analyses for unmeasured confounding assuming a marginal structural model for repeated measures. *Statistics in medicine* 23(5), 749–767.

Chen, S. X., D. H. Leung, and J. Qin (2008). Improving semiparametric estimation by using surrogate data. *Journal of the Royal Statistical Society: Series B* 70(4), 803–823.

Cole, S. R. and M. A. Hernán (2008). Constructing inverse probability weights for marginal structural models. *American Journal of Epidemiology* 168(6), 656–664.

Cox, D. R. (2006). *Principles of Statistical Inference*. Cambridge University Press.

Dawid, A. P. (1979). Conditional independence in statistical theory. *Journal of the Royal Statistical Society, Series B*, 1–31.

Deville, J.-C. and C.-E. Särndal (1992). Calibration estimators in survey sampling. *Journal of the American statistical Association* 87(418), 376–382.

Ding, P. and F. Li (2018). Causal inference: a missing data perspective. *Arxiv, preprint arXiv:1712.06170*.

Ertefaie, A. and D. A. Stephens (2010). Comparing approaches to causal inference for longitudinal data: Inverse probability weighting versus propensity scores. *The International Journal of Biostatistics* 6(2), 1–22.

Fuller, W. A., M. M. Loughin, and H. D. Baker (1994). Regression weighting for the 1987-88 national food consumption survey. *Survey Methodology* 20, 75–85.

Hernán, M. A. and J. M. Robins (2018). *Causal Inference*. Boca Raton: Chapman & Hall/CRC, forthcoming.

Hirano, K. and G. W. Imbens (2001). Estimation of causal effects using propensity score weighting: An application to data on right heart catheterization. *Health Services and Outcomes Research Methodology* 2(3-4), 259–278.
Hogan, J. W., J. Roy, and C. Korkontzelou (2004). Handling drop-out in longitudinal studies. *Statistics in Medicine* 23(9), 1455–1497.

Holland, P. W. (1986). Statistics and causal inference. *Journal of the American Statistical Association* 81(396), 945–960.

Hong, G. and S. W. Raudenbush (2006). Evaluating kindergarten retention policy: A case study of causal inference for multilevel observational data. *Journal of the American Statistical Association* 101(475), 901–910.

Horvitz, D. G. and D. J. Thompson (1952). A generalization of sampling without replacement from a finite universe. *Journal of the American Statistical Association* 47(260), 663–685.

Hosmer, D. W. and S. Lemeshow (2000). *Applied Logistic Regression*. John Wiley & Sons.

Imai, K. and M. Ratkovic (2014). Covariate balancing propensity score. *Journal of the Royal Statistical Society, Series B* 76(1), 243–263.

Imbens, G. W. and D. B. Rubin (2015). *Causal Inference for Statistics, Social, and Biomedical Sciences: An Introduction*. Cambridge University Press.

Kalton, G. and I. Flores-Cervantes (2003). Weighting methods. *Journal of Official Statistics* 19(2), 81.

Kim, J. K. and J. Im (2014). Propensity score adjustment with several follow-ups. *Biometrika* 101(2), 439–448.

Kim, J. K. and J. Shao (2013). *Statistical Methods for Handling Incomplete Data*. CRC Press.

Kott, P. S. and T. Chang (2010). Using calibration weighting to adjust for nonignorable unit nonresponse. *Journal of the American Statistical Association* 105(491), 1265–1275.
Li, F., A. M. Zaslavsky, and M. B. Landrum (2013). Propensity score weighting with multilevel data. *Statistics in Medicine* 32(19), 3373–3387.

Little, R. J. (1986). Survey nonresponse adjustments for estimates of means. *International Statistical Review/Revue Internationale de Statistique*, 139–157.

Liu, L., M. G. Hudgens, and S. Becker-Dreps (2016). On inverse probability-weighted estimators in the presence of interference. *Biometrika* 103(4), 829–842.

Lunceford, J. K. and M. Davidian (2004). Stratification and weighting via the propensity score in estimation of causal treatment effects: a comparative study. *Statistics in Medicine* 23(19), 2937–2960.

Miao, W., E. Tchetgen Tchetgen, and Z. Geng (2015). Identification and doubly robust estimation of data missing not at random with a shadow variable. *arXiv preprint arXiv:1509.02556*.

Mitra, R. and J. P. Reiter (2011). Estimating propensity scores with missing covariate data using general location mixture models. *Statistics in Medicine* 30(6), 627–641.

Naimi, A. I., E. E. Moodie, N. Auger, and J. S. Kaufman (2014). Constructing inverse probability weights for continuous exposures: a comparison of methods. *Epidemiology* 25(2), 292–299.

Neyman, J. (1923, 1990). On the application of probability theory to agricultural experiments. Essay on principles. Section 9. *Statistical Science* 5(4), 465–472.

Neyman, J. and E. L. Scott (1948). Consistent estimates based on partially consistent observations. *Econometrica: Journal of the Econometric Society* 16(1), 1–32.

O’Connor, A. M., S. D. Sorden, and M. D. Apley (2005). Association between the existence of calves persistently infected with bovine viral diarrhea virus and commingling on pen morbidity in feedlot cattle. *American Journal of Veterinary Research* 66(12), 2130–2134.
Ogburn, E. L., A. Rotnitzky, and J. M. Robins (2015). Doubly robust estimation of the local average treatment effect curve. *Journal of the Royal Statistical Society: Series B (Statistical Methodology)* 77(2), 373–396.

Pearl, J., M. Glymour, and N. P. Jewell (2016). *Causal Inference in Statistics: A Primer*. John Wiley & Sons.

Ramirez, A., C. Wang, J. R. Prickett, R. Pogranichniy, K.-J. Yoon, R. Main, J. K. Johnson, C. Rademacher, M. Hoogland, P. Hoffmann, et al. (2012). Efficient surveillance of pig populations using oral fluids. *Preventive Veterinary Medicine* 104(3-4), 292–300.

Robins, J. M., M. Á. Hernán, and B. Brumback (2000). Marginal structural models and causal inference in epidemiology. *Epidemiology* 11(5), 550–560.

Rosenbaum, P. R. and D. B. Rubin (1983). The central role of the propensity score in observational studies for causal effects. *Biometrika* 70(1), 41–55.

Rotnitzky, A. and J. M. Robins (1995). Semiparametric regression estimation in the presence of dependent censoring. *Biometrika* 82(4), 805–820.

Rubin, D. B. (1974). Estimating causal effects of treatments in randomized and nonrandomized studies. *Journal of Educational Psychology* 66(5), 688–701.

Simpson, E. H. (1951). The interpretation of interaction in contingency tables. *Journal of the Royal Statistical Society, Series B*, 238–241.

Sjölander, A., O. Nyrén, R. Bellocco, and M. Evans (2011). Comparing different strategies for timing of dialysis initiation through inverse probability weighting. *American Journal of Epidemiology* 174(10), 1204–1210.

Skinner, C. and J. D’arrigo (2011). Inverse probability weighting for clustered nonresponse. *Biometrika* 98(4), 953–966.
Sun, B. and E. J. Tchetgen Tchetgen (2017). On inverse probability weighting for nonmonotone missing at random data. *Journal of the American Statistical Association*, 1–11.

Tan, Z. (2010). Bounded, efficient and doubly robust estimation with inverse weighting. *Biometrika* 97(3), 661–682.

Tchetgen Tchetgen, E. J. and T. J. VanderWeele (2012). On causal inference in the presence of interference. *Statistical Methods in Medical Research* 21(1), 55–75.

Thoemmes, F. J. and S. G. West (2011). The use of propensity scores for nonrandomized designs with clustered data. *Multivariate Behavioral Research* 46(3), 514–543.

Tsiatis, A. (2006). *Semiparametric Theory and Missing Data*. Springer.

VanderWeele, T. J. (2009). Marginal structural models for the estimation of direct and indirect effects. *Epidemiology* 20(1), 18–26.

Vansteelandt, S. and R. M. Daniel (2014). On regression adjustment for the propensity score. *Statistics in Medicine* 33(23), 4053–4072.

Wen, L. and S. R. Seaman (2018). Semi-parametric methods of handling missing data in mortal cohorts under non-ignorable missingness. *Biometrics*.

Yang, S. (2017). Propensity score weighting for causal inference with clustered data. *Arxiv, preprint arXiv:1703.06086*.

Yuan, Y. and R. J. Little (2007). Model-based estimates of the finite population mean for two-stage cluster samples with unit non-response. *Journal of the Royal Statistical Society: Series C (Applied Statistics)* 56(1), 79–97.

Zhang, B., A. A. Tsiatis, E. B. Laber, and M. Davidian (2012). A robust method for estimating optimal treatment regimes. *Biometrics* 68(4), 1010–1018.
Zhao, Q., D. S. Small, and B. B. Bhattacharya (2017). Sensitivity analysis for inverse probability weighting estimators via the percentile bootstrap. *arXiv preprint arXiv:1711.11286*.

Zubizarreta, J. R. and L. Keele (2017). Optimal multilevel matching in clustered observational studies: A case study of the effectiveness of private schools under a large-scale voucher system. *Journal of the American Statistical Association 112*(518), 547–560.
Supplementary Materials

A Proof of Theorem 1

Proof. For any $z \in \Omega_Z$ and its corresponding subvector value $z_{\text{sub}} \in \Omega_{Z_{\text{sub}}}$, let $z^* \in \Omega_Z$ and its corresponding subvector value $z^*_{\text{sub}} \in \Omega_{Z_{\text{sub}}}$ satisfy (i) $z_{\text{sub}} \neq z^*_{\text{sub}}$ and (ii) $T(z) = T(z^*)$.
Because $T$ is sufficient for $\theta$, by Bayes rule,

$$P\{Z_{\text{sub}} = z_{\text{sub}} | X, T = T(z)\}$$

\[= P\{Z_{\text{sub}} = z_{\text{sub}} | X, \theta, T = T(z)\}\]

\[= \frac{P\{Z_{\text{sub}} = z_{\text{sub}}, T = T(z) | X, \theta\}}{P\{T = T(z) | X, \theta\}}.\]

Next we want to show the numerator of (14) is in range $(0,1)$. That is,

$$P\{Z_{\text{sub}} = z_{\text{sub}}, T = T(z) | X, \theta\} \geq P\{Z = z, Z_{\text{sub}} = z_{\text{sub}}, T = T(z) | X, \theta\} = P\{Z = z | X, \theta\} > 0,$$

$$P\{Z_{\text{sub}} = z_{\text{sub}}, T = T(z) | X, \theta\} \leq P\{Z_{\text{sub}} = z_{\text{sub}} | X, \theta\} < 1.$$

Therefore, $0 < P\{Z_{\text{sub}} = z_{\text{sub}}, T = T(z) | X, \theta\} < 1$. Last we want to show the denominator of (14) is greater than the numerator, which is

$$P\{T = T(z) | X, \theta\} \geq P\{Z_{\text{sub}} = z_{\text{sub}}, T = T(z) | X, \theta\} + P\{Z_{\text{sub}} = z^*_{\text{sub}}, T = T(z^*) | X, \theta\}$$

\[> P\{Z_{\text{sub}} = z_{\text{sub}}, T = T(z) | X, \theta\}.\]

So we can show that $0 < P\{Z_{\text{sub}} = z_{\text{sub}} | X, T = T(z)\} < 1$. \qed

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B Proof of Theorem 2

Proof. From Lemma 4.2 of Dawid (1979), we know that if $Z_1 \perp \perp Z_2 | Z_3, \theta$ and $T$ is a function of $Z_1$, then $T \perp \perp Z_2 | Z_3, \theta$ and $Z_1 \perp \perp Z_2 | Z_3, \theta, T$. Moreover, $T$ is sufficient for $\theta$, then $P(Z_1 | Z_3, T) = P(Z_1 | Z_3, T, \theta)$.

Let $f(\theta | Z_3, T)$ be the conditional density function for $\theta$ given $Z_3$ and $T$. If $Z_1 \perp \perp Z_2 | Z_3, \theta$, the joint probability for $Z_1$ and $Z_2$ conditional on $Z_3$ and $T$ is

\[
P(Z_1, Z_2 | Z_3, T) = \int_{\Omega_\theta} P(Z_1, Z_2, \theta | Z_3, T)f(\theta | Z_3, T)d\theta
\]

\[
= \int_{\Omega_\theta} P(Z_1, Z_2 | Z_3, T, \theta)f^2(\theta | Z_3, T)d\theta
\]

\[
= \int_{\Omega_\theta} P(Z_1 | Z_3, T, \theta)P(Z_2 | Z_3, T, \theta)f^2(\theta | Z_3, T)d\theta
\]

\[
= \int_{\Omega_\theta} P(Z_1 | Z_3, T)P(Z_2 | Z_3, T, \theta)f^2(\theta | Z_3, T)d\theta
\]

\[
= P(Z_1 | Z_3, T)\int_{\Omega_\theta} P(Z_2 | Z_3, T, \theta)f(\theta | Z_3, T)d\theta
\]

\[
= P(Z_1 | Z_3, T)P(Z_2 | Z_3, T).
\]

\[\square\]
C  Proof of Theorem 3

Proof. For treatment level $a$, we want to show the term $E\left\{ \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{I(A_{ij} = a)Y_{ij}}{P(A_{ij} = a|X_i, T_i; \beta)} \right\}$ is unbiased to $EY(a)$ and robust to both the correlation between $U_i$ and $X_i$ and the correlation between $U_i$ and $Y_i(a)$.

We treat $U_i$ as the cluster-specific parameter for all $i$. From Lemma 4.2 of Dawid (1979), $T_i$, a function of $A_i$, satisfies Assumption 5. We have $Y_i(a) \perp \perp A_i|X_i, T_i, U_i$ for all $i$ and $a$. Therefore,

$$E_{A_{ij}|x_i, T_i, U_i}\left\{ \frac{I(A_{ij} = a)}{P(A_{ij} = a|X_i, T_i; \beta)} X_i, T_i, U_i \right\} = E_{A_{ij}|x_i, T_i, U_i}\left\{ \frac{I(A_{ij} = a)}{P(A_{ij} = a|X_i, T_i; \beta)} X_i, T_i, U_i \right\} = 1.$$

$$E\left\{ \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{I(A_{ij} = a)Y_{ij}}{P(A_{ij} = a|X_i, T_i; \beta)} \right\}$$

$$= \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} E_{A_{ij}, Y_{ij}(a)}\left\{ \frac{I(A_{ij} = a)}{P(A_{ij} = a|X_i, T_i; \beta)} Y_{ij}(a) \right\}$$

$$= \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} E_{X_i, T_i, U_i}\left[ E_{A_{ij}, Y_{ij}(a)|X_i, T_i, U_i}\left\{ \frac{I(A_{ij} = a)}{P(A_{ij} = a|X_i, T_i, U_i)} Y_{ij}(a) \right\} X_i, T_i, U_i \right]$$

$$= \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} E_{X_i, T_i, U_i}\left[ E_{Y_{ij}(a)|X_i, T_i, U_i}\{Y_{ij}(a)|X_i, T_i, U_i\} \right]$$

$$= \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} E_{Y_{ij}(a)}[Y_{ij}(a)]$$

$$= E[Y(a)].$$

The above equation holds due to Assumptions 2 and 4*. Therefore, we can prove Theorem 3. $\square$
D Conditions for the Asymptotic Properties of CMLE of $\beta$

In Andersen (1970), $\beta$ is called a structural parameter and $b$’s are called incidental parameters. In our case, we treat $\{U_i\}_{i=1}^m$ as the incidental parameters and $U_i \in \Omega_U$, which is compact. Moreover, $n_i$ is fixed and bounded. The following conditions are adopted from those in Andersen (1970).

**Condition 1:** The log density function $\log \phi_{ij}(a|x_i, t_i; \beta)$ is a differentiable function of $\beta$ for all $i$ and $j$, and there exists a set $B$ of values of $t$ with $P_{\beta_0, U}(T^{-1}B) > 0$ for all $U \in \Omega_U$ and an open set $\Omega_0$ containing the true value of the parameter $\beta_0$ such that for any minimal sufficient statistic $t \in B$ and $x \in \Omega_X$, where $\Omega_X$ is compact, the functions $\phi_{ij}(a|x, t; \beta)$ and $\phi_{ij}(a|x, t; \beta')$ are not identical for any pair $\beta \in \Omega_0$ and $\beta' \in \Omega_0$.

**Condition 2:** The maximum likelihood estimating equation

$$\sum_{i=1}^m \sum_{j=1}^{n_i} \{\partial \log \phi_{ij}(a_{ij}|x_i, t_i; \beta) / \partial \beta \} = 0$$

has a unique solution $\hat{\beta}_n \in \Omega_\beta$, which is compact, for almost all values of the vector $(t_1, \cdots, t_m)$.

**Condition 3:** For all $\delta \in \Delta$, where $\Delta$ is an open set containing $0$,

$$\sum_{i=1}^{\infty} \sigma^2(\delta, U_i)/i^2 < \infty,$$

where $\sigma^2(\delta, U_i) = var_{\beta_0, U_i}\{\sum_{j=1}^{n_i} \log \phi_{ij}(A_{ij}|X_i, T_i; \beta_0 + \delta) - \sum_{j=1}^{n_i} \log \phi_{ij}(A_{ij}|X_i, T_i; \beta_0)\}$ for all $U_i \in \Omega_U$.

**Condition 4:** The set of first, second, and third partial derivatives of cluster-level log joint density function $\sum_{j=1}^{n_i} \log \phi_j(a|X, T; \beta)$ exist for all $\beta$ in an open set $\Omega_0$ enclosing $\beta_0$.

Let

$$B_2(\beta) = \frac{1}{n} \sum_{i=1}^m \sum_{j=1}^{n_i} E\left\{\frac{\partial}{\partial \beta \partial \beta^T} \log \phi_{ij}(A_{ij}|X_i, T_i; \beta)\right\}$$
and
\[ B_3(\beta) = \frac{1}{n} \sum_{i=1}^{m} E \left\{ \sum_{j=1}^{n} \frac{\partial}{\partial \beta} \log \phi_{ij}(A_{ij}|X_i, T_i; \beta) \right\} \left\{ \sum_{j=1}^{n} \frac{\partial^2}{\partial \beta^2} \log \phi_{ij}(A_{ij}|X_i, T_i; \beta) \right\}. \]

For all \( U \in \Omega_U \) and all \( \beta \in \Omega_0 \), we have
\[ E_{\beta,U} \left\{ \sum_{j=1}^{n} \frac{\partial\log \phi_j(A_j|X, T; \beta)}{\partial \beta_k} \right\} = 0. \]

for \( k = 1, \cdots, p \). There further exist positive integrable functions \( c_{kl}(a_1, \cdots, a_{n_i}) \) such that
\[ |\sum_{j=1}^{n} \frac{\partial^3 \log \phi_j(a_j|X, T; \beta)}{(\partial \beta_k \partial \beta_l \partial \beta_q)}| \leq c_{kl}(a_1, \cdots, a_{n_i}) \]

for \( \beta \in \Omega_0 \) and \( q = 1, \cdots, p \), and such that \( E_{\beta_0,U} c_{kl}(A_1, \cdots, A_{n_i}) \) and \( \text{var}_{\beta_0,U} c_{kl}(A_1, \cdots, A_{n_i}) \) are continuous.

Condition 5: For all \( a \), the density function \( f(a|\beta_0, U) \) is a continuous function of all \( U \in \Omega_U \) and for \( k, l = 1, \cdots, p \), \( \text{var}_{\beta_0,U} \left\{ \sum_{j=1}^{n} \frac{\partial^2 \log \phi_j(A_j|X, T; \beta)}{(\partial \beta_k \partial \beta_l)} \right\} \) and \( E_{\beta_0,U} \left\{ \sum_{j=1}^{n} \frac{\partial \log \phi_{ij}(A_{ij}|X_i, T_i; \beta)}{\partial \beta_k} \right\} \left\{ \sum_{j=1}^{n} \frac{\partial \log \phi_{ij}(A_{ij}|X_i, T_i; \beta)}{\partial \beta_l^T} \right\} \) are continuous of \( U \). In addition, the matrix \( B_2(\beta) \) is non-singular.
E Proof of Theorem 6

Proof. For notation convenience, let

\[
\hat{Y}_{ICPW}(a) := \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{\mathcal{I}(A_{ij} = a) Y_{ij}}{P(A_{ij} = a|X_i, T_i; \beta)} = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \phi_{ij}(A_{ij}|X_i, T_i; \beta),
\]

\[
\hat{Y}_{\beta_0}(a) := \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{\mathcal{I}(A_{ij} = a) Y_{ij}}{P(A_{ij} = a|X_i, T_i; \beta_0)} = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \phi_{ij}(A_{ij}|X_i, T_i; \beta_0).
\]

We have

\[
\sqrt{n}[\hat{Y}_{ICPW}(a) - E\{Y(a)\}] = \sqrt{n}[\hat{Y}_{n}(\hat{\beta}; a) - E\{Y(a)\}]
\]

\[
= \sqrt{n}[\hat{Y}_{n}(\hat{\beta}; a) - \hat{Y}_{n}(\beta_0; a)] + \sqrt{n}[\hat{Y}_{n}(\beta_0; a) - E\{Y(a)\}].
\]

By Chebyshev’s inequality, we can show that

\[
P\{\sqrt{n}[\hat{Y}_{n}(\beta_0; a) - E\{Y(a)\}] > n\} < \frac{\sigma^2_{\beta_0}}{n}.
\]

In other words, \(\sqrt{n}[\hat{Y}_{n}(\beta_0; a) - E\{Y(a)\}] \to 0\) in probability when \(n \to \infty\). By Delta method with Taylor series expansion,

\[
\hat{Y}_{n}(\hat{\beta}; a) = \hat{Y}_{n}(\beta_0; a) + \frac{\partial \hat{Y}_{n}(\beta; a)}{\partial \beta} \bigg|_{\beta=\beta_0} (\hat{\beta} - \beta_0) + o_p(1)
\]

\[
= \hat{Y}_{n}(\beta_0; a) - \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{\mathcal{I}(A_{ij} = a) Y_{ij}}{\partial \phi_{ij}(a|X_i, T_i; \beta)} \bigg|_{\beta=\beta_0} (\hat{\beta} - \beta_0) + o_p(1).
\]

Therefore, we have

\[
\sqrt{n}\{\hat{Y}_{ICPW}(a) - \hat{Y}_{n}(\beta_0; a)\} \to N(0, V_1(\beta_0))
\]
in distribution as \( n \to \infty \), where \( V_1(\beta_0) = E[H_1(\beta_0)^T B_1(\beta_0) H_1(\beta_0)] \), and

\[
H_1(\beta) = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \frac{I\{A_{ij} = a\} Y_{ij} \partial \phi_{ij}(a|X_i, T_i; \beta)}{\phi_{ij}^2(a|X_i, T_i; \beta)} \partial \beta.
\]

By Slutsky’s theorem, we conclude that

\[
\sqrt{n} V_1(\beta_0)^{-1/2}(\tilde{Y}_{ICPW}(a) - E\{Y(a)\}) \to N(0,1).
\]

\(\blacksquare\)
F Proof of Theorem 7

Proof. For notation convenience, let

\[
\hat{\tau}_n(\hat{\beta}) := \hat{\tau}_{ICPW}
\]

\[
= \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n} \left\{ \frac{A_{ij} Y_{ij}}{P(A_{ij} = 1|X_i, T_i; \beta)} - \frac{(1 - A_{ij}) Y_{ij}}{1 - P(A_{ij} = 1|X_i, T_i; \beta)} \right\}
\]

\[
= \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n} \left\{ \frac{A_{ij} Y_{ij}}{\phi_{ij}(\beta)} - \frac{(1 - A_{ij}) Y_{ij}}{1 - \phi_{ij}(\beta)} \right\}.
\]

\[
\hat{\tau}_n(\beta_0) := \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n} \left\{ \frac{A_{ij} Y_{ij}}{P(A_{ij} = 1|X_i, T_i; \beta_0)} - \frac{(1 - A_{ij}) Y_{ij}}{1 - P(A_{ij} = 1|X_i, T_i; \beta_0)} \right\}
\]

\[
= \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n} \left\{ \frac{A_{ij} Y_{ij}}{\phi_{ij}(\beta_0)} - \frac{(1 - A_{ij}) Y_{ij}}{1 - \phi_{ij}(\beta_0)} \right\}.
\]

We have

\[
\sqrt{n}(\hat{\tau}_{ICPW} - \tau) = \sqrt{n}[\hat{\tau}_n(\hat{\beta}) - \hat{\tau}_n(\beta_0)] + \sqrt{n}[\hat{\tau}_n(\beta_0) - \tau].
\]

By Chebyshev’s inequality, we can show that

\[
P\{|\sqrt{n}[\hat{\tau}_n(\beta) - \tau]| > n\} < \frac{\sigma^2_{\hat{\tau}_n(\beta_0)}}{n}.
\]

In other words, \(\sqrt{n}[\hat{\tau}_n(\beta_0) - \tau] \to 0\) in probability when \(n \to \infty\). By Delta method with Taylor series expansion,

\[
\hat{\tau}_n(\hat{\beta}) = \hat{\tau}_n(\beta_0) + \left. \frac{\partial \hat{\tau}_n(\beta)}{\partial \beta} \right|_{\beta = \beta_0} (\hat{\beta} - \beta_0) + o_p(1)
\]

\[
= \hat{\tau}_n(\beta_0) - \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n} \left\{ \frac{A_{ij} Y_{ij}}{\phi^2_{ij}(\beta_0)} + \frac{(1 - A_{ij}) Y_{ij}}{(1 - \phi_{ij}(\beta_0))^2} \right\} \frac{\partial \phi_{ij}(\beta)}{\partial \beta} \bigg|_{\beta = \beta_0} (\hat{\beta} - \beta_0) + o_p(1).
\]
Therefore, we have

$$\sqrt{n}\{\hat{\tau}_{ICPW} - \hat{\tau}_n(\beta_0)\} \to N(0, V_2(\beta_0))$$

in distribution as $n \to \infty$, where $V_2(\beta_0) = E[H_2(\beta_0)^T B_1(\beta_0) H_2(\beta_0)]$, and

$$H_2(\beta) = \frac{1}{n} \sum_{i=1}^{m} \sum_{j=1}^{n_i} \left\{ \frac{A_{ij}}{\phi_{ij}^2(\beta)} + \frac{1 - A_{ij}}{|1 - \phi_{ij}(\beta)|^2} \right\} Y_{ij} \frac{\partial \phi_{ij}(\beta)}{\partial \beta}.$$

By Slutsky’s theorem, we conclude that

$$\sqrt{n}V_2(\beta_0)^{-1/2}(\hat{\tau}_{ICPW} - \tau) \to N(0, 1).$$

$\square$