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## WIN RATIO FOR PARTIALLY ORDERED DATA

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*Abstract:* The win ratio, initially developed for time-to-event data, can be extended to any data type equipped with a partial order. We study this extension in both nonparametric inference and semiparametric regression. We begin by formulating the win ratio as an estimand of contrast between two populations with partially ordered responses, showing that it reduces to the familiar odds ratio in the case of binary data. For hypothesis testing, we prove that the empirical two-sample win ratio is consistent against stochastically ordered distributions and efficient against proportional odds alternatives under a total order. In regression, we model the conditional win ratio multiplicatively against covariates, extending logistic regression from binary to partially ordered responses. This model is implied by a generalized continuation-ratio logit model but requires fewer assumptions on the relationship between response levels. To make inference, we construct a class of weighted  $U$ -statistic estimating equations and derive pseudo-efficient weights to improve efficiency. Simulation studies demonstrate that the proposed procedures perform well in both testing and regression under

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finite samples. As illustrations, we analyze bivariate radiologic assessments in a recent liver disease study and subject smoking status in a youth tobacco use study, treating them both as partially ordered outcomes. The proposed methodology is implemented in the R package `poset`, publicly available on GitHub at <https://lmaowisc.github.io/poset> and on the Comprehensive R Archive Network (CRAN).

*Key words and phrases:* Continuation ratio; Logistic regression; Odds ratio; Ordinal data; Stochastic order;  $U$ -statistic.

## 1. Introduction

Partially ordered data, a common variant of ordinal data, frequently arise in medical and sociological studies. Unlike totally ordered tumor grades or Likert scales, partially ordered data are not necessarily pairwise comparable. For instance, in the tumor-node-metastasis cancer staging system (Edge et al., 2010), primary and metastatic tumors are scored on four- and three-level severity scales, respectively. Two patients can be compared in overall severity only if one scores higher on both scales (Lin et al., 2013). Similar partial orders are found in groups of radiologic ratings or survey responses.

Despite their prevalence, partially ordered data have received only limited methodological attention. In the nonparametric setting, Rosenbaum (1991) discussed desirable properties for statistics measuring the association

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between two partially ordered responses (where one may be a binary treatment). He demonstrated that the Wilcoxon rank sum and Spearman's rank correlation statistics satisfy these properties in the special case of totally ordered data. Mondal & Hinrichs (2016) proposed a class of association tests by conceptualizing an underlying total order consistent with the partial order and then computing rank statistics based on that latent order. However, the existence of such a compatible total order is not guaranteed, and a direct measure of between-group difference is not provided.

For regression analysis, Zhang & Ip (2012) proposed a class of partitioned conditional models, constructed in three consecutive steps. First, a nominal (i.e., multinomial) regression model is used to estimate the probabilities of any disjoint networks across which no two elements are comparable (if more than one such network exist). Next, each network is partitioned into a sequence of weakly ordered antichains (Trotter, 1992), i.e., subsets containing mutually incomparable elements, whose conditional probabilities are modeled using ordinal regression, such as the proportional odds model (McCullagh, 1980; Agresti, 2010). Finally, the mutually incomparable elements within each antichain are modeled using nominal regression. Peyhardi et al. (2016) extended this approach to general categorical responses. Although the partitioned conditional models elegantly reduce complex par-

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tial orders into layers of familiar ordinal or nominal structures, they involve extensive model assumptions that are difficult to verify. Moreover, the large number of regression coefficients, many of which are not of direct interest, complicates the interpretation of covariate effects.

A potential solution lies in the win ratio, which has recently gained popularity in the analysis of composite time-to-event outcomes (Pocock et al., 2012). Originally, the win ratio was defined as the fraction of wins to losses among all pairs between treatment and control, based on a specific comparison rule. This concept is well-suited for partially ordered data, with the partial order serving as the comparison rule and incomparable pairs treated as “ties”. Unlike the elaborate partitioned conditional models, the win ratio focuses on dimensions with a clear ordering between outcomes, providing a succinct summary of treatment effects. Even for totally ordered outcomes, the win ratio may offer a more robust alternative to standard ordinal regression (Agresti, 2010), as it imposes no constraints on the relationships between response levels. While the two-sample win ratio has been informally suggested for partially ordered data (Wittkowski et al., 2004; Bebu & Lachin, 2016), its statistical properties as estimators and tests remain unexplored. Additionally, regression modeling of partially ordered data using the win ratio has yet to be investigated in the literature.

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In this work, we conduct a detailed study of the win ratio for partially ordered data in both nonparametric inference and regression analysis. Specifically, we define the win ratio formally as a model-free estimand and explore the operating characteristics of its empirical estimator in two-sample testing. Quantitative covariates are incorporated via a multiplicative model, in which the regression coefficients are interpreted as the log-win ratios resulting from unit increases in the covariates. Estimating functions are constructed based on covariate-weighted pairwise residuals, with efficient weights informed by parallels with logistic regression and its well-known efficient score function.

## 2. Two-sample estimation and testing

### 2.1 Set-up

Let  $(\mathcal{Y}, \preceq)$  denote a partially ordered set, or poset (Trotter, 1992), where the set  $\mathcal{Y}$  is equipped with a partial order  $\preceq$ . Throughout, we assume that  $\mathcal{Y}$  is discrete with at most countably many elements. The partial order  $\preceq$  is a relation between pairs of elements in  $\mathcal{Y}$  that satisfies the following properties: it is reflexive ( $y \preceq y$  for all  $y \in \mathcal{Y}$ ), anti-symmetric ( $y \preceq y^*$  and  $y^* \preceq y$  imply  $y = y^*$ ), and transitive ( $y \preceq y^*$  and  $y^* \preceq y^{**}$  imply  $y \preceq y^{**}$ , for all  $y, y^*,$  and  $y^{**} \in \mathcal{Y}$ ). Occasionally, the relation  $y \preceq y^*$  will

be equivalently expressed as  $y^* \succeq y$ . If neither  $y \preceq y^*$  nor  $y^* \preceq y$ , the pair is said to be incomparable. If every pair in  $\mathcal{Y}$  is comparable, the poset is said to be totally ordered.

**Example 1** (Ordinal data). Ordinal data, such as tumor grades and Likert scales, are totally ordered. If  $\mathcal{Y}$  consists of  $m$  totally ordered elements, we can, without loss of generality, let  $(\mathcal{Y}, \preceq) = (\mathbb{N}_{m-1}, \leq)$ , where  $\mathbb{N}_{m-1} = \{0, 1, \dots, m-1\}$ . Binary data are a special case with  $m = 2$ .

**Example 2** (Multivariate ordinal data). A more complex data type arises when multiple ordinal attributes are combined, with two observations being comparable if and only if the same order holds across all components. In poset terminology, this is referred to as the product order (Garg, 2015). Alternatively, certain components may be prioritized so that other components are compared only when the prioritized ones are tied. This is known as a lexicographic order (similar to how words are arranged in a dictionary) (Garg, 2015). In general, the poset for outcomes with  $K$  ordinal components can be represented by  $(\mathcal{Y}, \preceq) = (\prod_{k=1}^K \mathbb{N}_{m_k-1}, \preceq)$ , where  $\prod_{k=1}^K \mathbb{N}_{m_k-1} = \mathbb{N}_{m_1-1} \times \dots \times \mathbb{N}_{m_K-1}$  (assuming the  $k$ th component has  $m_k$  levels). In the case of a product order,  $\preceq$  can be replaced by  $\leq$ , which is understood to operate component-wise.

Every partial order  $\preceq$  induces a corresponding strict partial order  $\prec$ .

Namely, if  $y \preceq y^*$  and  $y \neq y^*$ , then we say  $y \prec y^*$ , or equivalently,  $y^* \succ y$ . By definition, the strict order is irreflexive, i.e.,  $y \not\prec y$  for all  $y \in \mathcal{Y}$ , and transitive. In Example 1, the strict order  $\prec$  is simply the strict inequality  $<$ ; in Example 2 with the product order,  $y_i \prec y_j$  if  $y_i \leq y_j$  component-wise with strict inequality for at least one component. Without loss of generality, we use the symbol  $\succ$  as the win operator; that is, we say that  $y_i$  wins against  $y_j$  if  $y_i \succ y_j$ . In both Examples, this means a higher-numbered outcome is more favorable.

Given  $y \in \mathcal{Y}$ , the upper and lower closures of  $y$  are defined by  $U[y] = \{y^* \in \mathcal{Y} : y^* \succeq y\}$  and  $D[y] = \{y^* \in \mathcal{Y} : y^* \preceq y\}$ , respectively. Similarly, the strict upper and lower closures are defined by  $U(y) = \{y^* \in \mathcal{Y} : y^* \succ y\}$  and  $D(y) = \{y^* \in \mathcal{Y} : y^* \prec y\}$ , respectively. The greatest or least element of a subset  $A \subset \mathcal{Y}$ , if it exists, is defined as the unique element  $s \in A$  such that  $s^* \preceq s$  or  $s^* \succeq s$ , respectively, for all  $s^* \in A$ . A lattice is a special type of poset such that, for any two elements  $y_i$  and  $y_j$ , a least element exists for  $U[y_i] \cap U[y_j]$  and a greatest element exists for  $D[y_i] \cap D[y_j]$  (Garg, 2015). It is easy to see that the data spaces described in Examples 1 and 2 are both lattices. Non-lattice posets are also common in applications. For instance, Zhang & Ip (2012) described a national longitudinal study on youth tobacco use with six levels of smoking behavior ordered in a non-lattice structure.



## 2.2 Nonparametric estimand and estimator

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For a probability measure  $\nu$  on  $\mathcal{Y}$ , we define some shorthand notation as follows. With a random element  $Y \sim \nu$ , denote  $\nu(y) = \text{pr}(Y = y)$  for  $y \in \mathcal{Y}$ ,  $\nu(A) = \text{pr}(Y \in A)$  for  $A \subset \mathcal{Y}$ , and  $\nu(f) = E\{f(Y)\}$  for an integrable function  $f : \mathcal{Y} \rightarrow \mathbb{R}$ . For every  $\nu$  in question, it is always assumed that  $\nu$  is supported on  $\mathcal{Y}$ , i.e.,  $\nu(y) > 0$  for all  $y \in \mathcal{Y}$ .

### 2.2 Nonparametric estimand and estimator

Let  $\nu_1$  and  $\nu_0$  denote the distributions of a partially ordered outcome in the treatment and control groups, respectively. An expedient way to compare the two groups is to assign a numeric score to each outcome level and consider the difference in the average score. More formally, let  $f : \mathcal{Y} \rightarrow \mathbb{R}$  be a non-decreasing function in the sense that  $y_i \preceq y_j$  implies  $f(y_i) \leq f(y_j)$ . The treatment effect can then be measured by  $\nu_1(f) - \nu_0(f)$ . Alternatively, one could model the relationship between  $\nu_1$  and  $\nu_0$  parametrically, for example using the proportional odds model in the case of ordinal outcomes, and then evaluate the model-based effect size, such as the odds ratio.

The win ratio as a nonparametric estimand does not rely on such arbitrary scoring or modeling. Define it as

$$\mathcal{R}(\nu_1, \nu_0) = \frac{\mathcal{W}(\nu_1, \nu_0)}{\mathcal{W}(\nu_0, \nu_1)} \equiv \frac{\int \int I(y_1 \succ y_0) \nu_1(dy_1) \nu_0(dy_0)}{\int \int I(y_0 \succ y_1) \nu_1(dy_1) \nu_0(dy_0)}, \quad (2.1)$$

where  $I(\cdot)$  is the indicator function. If  $Y_z \sim \nu_z$  ( $z = 1, 0$ ) and  $Y_1 \perp\!\!\!\perp Y_0$ ,

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2.2 Nonparametric estimand and estimator

we have  $\mathcal{W}(\nu_z, \nu_{1-z}) = \text{pr}(Y_z \succ Y_{1-z})$ , which represents the probability of group  $z$  winning against  $1-z$ . Hence, the ratio  $\mathcal{R}(\nu_1, \nu_0)$  can be interpreted as the fold change in the likelihood of winning by the treatment compared to the control. This interpretation is similar to that of the odds ratio for a binary outcome. In fact, the metric reduces to the odds ratio in the binary case.

**Proposition 1** (Equivalence of win and odds ratios). *For  $(\mathbb{N}_1, \leq)$ ,*

$$\mathcal{R}(\nu_1, \nu_0) = \frac{\nu_1(1)\{1 - \nu_0(1)\}}{\nu_0(1)\{1 - \nu_1(1)\}}.$$

*Proof.* The result follows by  $\mathcal{W}(\nu_z, \nu_{1-z}) = \nu_z(1)\nu_{1-z}(0)$  ( $z = 1, 0$ ).  $\square$

Let  $\hat{\nu}_z$  denote the empirical distribution in group  $z$  of size  $n_z$  ( $z = 1, 0$ ). Then, a natural estimator for  $\mathcal{R}(\nu_1, \nu_0)$  is the plug-in  $\mathcal{R}(\hat{\nu}_1, \hat{\nu}_0)$ , which is just a compact notation for the empirical win ratio statistic based on pairwise comparisons. It then follows that  $\mathcal{R}(\hat{\nu}_1, \hat{\nu}_0)$  is an efficient estimator for  $\mathcal{R}(\nu_1, \nu_0)$  due to the efficiency of the empirical distribution functions and the efficiency-preservation property of  $\mathcal{R}(\cdot, \cdot)$  as a smooth functional (Bickel et al., 1993). Like the Wilcoxon rank sum statistic,  $\mathcal{R}(\hat{\nu}_1, \hat{\nu}_0)$  also satisfies the decreasing reflection property of Rosenbaum (1991) for association metrics between an ordered outcome and a binary treatment. In fact, if we think of the  $\mathcal{W}(\hat{\nu}_z, \hat{\nu}_{1-z})$ , i.e., the empirical win and loss proportions, as the partial-

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### 2.3 Operating characteristics of the test

order equivalent of ranks, we can view the win ratio as an extension of the Wilcoxon rank statistic (Mao, 2022). Finally, the asymptotic normality of  $\mathcal{R}(\hat{\nu}_1, \hat{\nu}_0)$  and its variance are derived in the supplementary material, using similar  $U$ -statistic techniques to those used for time-to-event outcomes (Luo, 2015; Bebu & Lachin, 2016; Dong et al., 2016). The results can also be obtained as a special case of the multiplicative regression model described in Section 3, with the treatment indicator as the only covariate.

### 2.3 Operating characteristics of the test

In addition to measuring treatment effect,  $\mathcal{R}(\hat{\nu}_1, \hat{\nu}_0)$  can also be used to test the null hypothesis  $H_0 : \nu_1 = \nu_0$ . This test will be particularly sensitive if  $\nu_0$  and  $\nu_1$  are stochastically ordered.

**Definition 1** (Stochastic order). A subset  $B \subset \mathcal{Y}$  is called an up-set if  $y \in B$  implies  $U[y] \subset B$ . Given two probability measures  $\nu_0$  and  $\nu_1$  on  $\mathcal{Y}$ ,  $\nu_1$  is stochastically greater than  $\nu_0$ , denoted by  $\nu_0 \preceq \nu_1$ , or  $\nu_1 \succeq \nu_0$ , if

$$\nu_1(B) \geq \nu_0(B) \text{ for every up-set } B \subset \mathcal{Y}.$$

This definition extends the concept of stochastic order from Euclidean spaces to posets, capturing the intuitive notion that one random element “tends” to win against another. An important implication of  $\nu_1 \succeq \nu_0$  is

### 2.3 Operating characteristics of the test

that  $\nu_1(f) \geq \nu_0(f)$  for every non-decreasing function  $f$  (Kamae & Krengel, 1978). An example of such a function on  $(\prod_{k=1}^K \mathbb{N}_{m_k-1}, \leq)$  is the average score  $f(y) = K^{-1} \sum_{k=1}^K s_{ki_k}$ , where  $y = (i_1, \dots, i_K)^\top$  and  $s_{k0} \leq s_{k1} \leq \dots \leq s_{k, m_k-1}$  is an ordered sequence of scores assigned to the  $m_k$  levels of the  $k$ th component ( $k = 1, \dots, K$ ).

Write  $n = n_1 + n_0$  and assume that  $n_1/n \rightarrow q \in (0, 1)$  as  $n \rightarrow \infty$ . By derivation in the supplementary material, we find that the log-transformed and normalized test statistic  $S_n = \hat{\sigma}_n^{-1} n^{1/2} \log\{\mathcal{R}(\hat{\nu}_1, \hat{\nu}_0)\} \rightarrow_d N(0, 1)$  under  $H_0$ , where  $\hat{\sigma}_n^2$  is a nonparametric variance estimator described in the supplementary material. A two-sided, asymptotic level- $\alpha$  test rejects  $H_0$  if  $|S_n| > z_{1-\alpha/2}$ , where  $z_{1-\alpha/2}$  is the  $(1 - \alpha/2)$ th quantile of the standard normal distribution.

We show that this test is consistent against the alternative hypothesis  $H_A : \nu_1 \succ \nu_0$ , i.e.,  $\nu_1 \succeq \nu_0$  but  $\nu_1 \neq \nu_0$ . For a nontrivial poset  $\mathcal{Y}$ , the asymptotic variance of  $n^{1/2} \log\{\mathcal{R}(\hat{\nu}_1, \hat{\nu}_0)\}$  is finite, so the noncentrality parameter for  $S_n$  is of the order  $O[n^{1/2} \log\{\mathcal{R}(\nu_1, \nu_0)\}]$ . As a result, the rejection probability tends to 1 as  $n \rightarrow \infty$  if  $\mathcal{R}(\nu_1, \nu_0) > 1$ . The following lemma establishes an intermediate result for showing that  $\mathcal{R}(\nu_1, \nu_0) > 1$  under  $\nu_1 \succ \nu_0$ . The proof can be found in the Appendix.

## 2.3 Operating characteristics of the test

**Lemma 1.** *If  $\nu_1 \succ \nu_0$ , then*

$$\nu_1\{U(y)\} \geq \nu_0\{U(y)\} \text{ for all } y \in \mathcal{Y},$$

*with strict inequality for at least one  $y$ .*

In addition to consistency, we can also show that the win ratio test, when applied to ordinal data, is asymptotically efficient under proportional odds alternatives.

**Theorem 1.** *The win ratio test has the following properties.*

(a) *When  $\nu_1 \succ \nu_0$ , for every  $\alpha \in (0, 1)$ ,*

$$\text{pr}(|S_n| > z_{1-\alpha/2}) \rightarrow 1 \text{ as } n \rightarrow \infty.$$

(b) *With a totally ordered  $\mathcal{Y}$ , when  $\nu_1$  and  $\nu_0$  conform to a proportional odds model with a non-unit odds ratio, the win ratio test achieves the highest asymptotic power possible. The precise statement is given in the supplementary material.*

*Proof.* To prove (a), by earlier arguments it suffices to show that  $\mathcal{W}(\nu_1, \nu_0) > \mathcal{W}(\nu_0, \nu_1)$ . But

$$\begin{aligned} \mathcal{W}(\nu_1, \nu_0) &= \int \nu_1\{U(y)\}\nu_0(dy) > \int \nu_0\{U(y)\}\nu_0(dy) \\ &\geq \int \nu_0\{U(y)\}\nu_1(dy) = \mathcal{W}(\nu_0, \nu_1), \end{aligned}$$

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where the first equality follows by Lemma 1 and the second by  $\nu_1 \succeq \nu_0$  and  $\nu_0\{U(y)\}$  being a non-increasing function of  $y$ . The proof of (b) is more involved and is relegated to the supplementary material, where the asymptotic power function is derived explicitly.  $\square$

### 3. Regression analysis

#### 3.1 A multiplicative win ratio model

With a  $p$ -dimensional covariate  $Z \in \mathbb{R}^p$ , we aim to assess its effects on  $Y \in \mathcal{Y}$ . Like in the two-sample case, direct modeling of the conditional distribution, denoted by  $\nu(\cdot | Z)$ , would likely involve unnecessary assumptions and nuisance parameters non-essential to the covariate effects. In keeping with the two-group win ratio defined in (2.1), we focus on the covariate-specific win ratio  $\mathcal{R}\{\nu(\cdot | Z_i), \nu(\cdot | Z_j)\}$ , where  $Z_i$  and  $Z_j$  are independent copies of  $Z$ . For convenience, we denote this quantity as  $\mathcal{R}(Z_i, Z_j)$ . Fixing  $Z_i$  and  $Z_j$ ,  $\mathcal{R}(Z_i, Z_j)$  is conceptually indistinguishable from the two-group win ratio studied in Section 2.2, only with the groups redefined as the subpopulations with covariates  $Z_i$  and  $Z_j$ . Specifically, if  $Y_i$  and  $Y_j$  are outcomes sampled independently from covariate groups  $Z_i$  and  $Z_j$ , respectively, then  $\mathcal{R}(Z_i, Z_j) = \text{pr}(Y_i \succ Y_j | Z_i, Z_j) / \text{pr}(Y_j \succ Y_i | Z_i, Z_j)$ , which represents the fold change in the likelihood of winning for subpopulation  $Z_i$

### 3.1 A multiplicative win ratio model

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as compared to  $Z_j$ .

With multi-dimensional  $Z$ , especially when it contains continuous components, a parsimonious model for  $\mathcal{R}(Z_i, Z_j)$  is desired to avoid the curse of dimensionality. Since  $\mathcal{R}(\cdot, \cdot) \in \mathbb{R}^+$  and must satisfy  $\mathcal{R}(Z_i, Z_j)\mathcal{R}(Z_j, Z_i) \equiv 1$ , it is natural to consider

$$\log\{\mathcal{R}(Z_i, Z_j)\} = \beta^T(Z_i - Z_j), \quad (3.2)$$

where  $\beta$  is a  $p$ -dimensional vector of regression coefficients. The components of  $\beta$  are thus the log-win ratios resulting from unit increases in the corresponding components of  $Z$ . If  $Z$  contains dummy variables encoding a categorical covariate, then (3.2) becomes a saturated model with  $\exp(\beta)$  containing the nonparametric win ratios, as defined in Section 2.2, comparing each level with the reference.

In general, model (3.2) implies that the covariates have multiplicative effects on the win ratio and will hence be called the multiplicative win ratio regression model. It is semiparametric in the sense that, apart from the parameterized conditional win ratio, other aspects of the conditional distribution of  $Y$  given  $Z$  are left unspecified.

**Remark 1.** Model (3.2) is similar in structure and interpretation to the proportional win-fractions model of Mao & Wang (2021) for composite time-to-event outcomes. The latter, however, involves stricter assumptions on

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### 3.2 Model-generating mechanisms

the constancy of the win ratio as follow-up goes on (hence the proportionality). If instead one chooses to model the win ratio under a fixed time frame, then the (possibly component-prioritized) composite outcomes would likely follow a partial order, and model (3.2) would apply.

### 3.2 Model-generating mechanisms

While model (3.2) allows for a simple and intuitive interpretation of covariate effects, its pairwise formulation hides the underlying response-covariate relationship. For transparency, we explore the possible scenarios under which (3.2) holds.

First, we show that under  $(\mathbb{N}_1, \leq)$ , model (3.2) is equivalent to the standard logistic regression with the same regression coefficients. In this case,  $\beta$  in (3.2) can be interpreted as either the log-win ratio or log-odds ratio, extending the two-sample equivalence result in Proposition 1 to regression models. The proof is straightforward and can be found in the Appendix.

**Proposition 2** (Equivalence with logistic regression for binary data). *For  $(\mathbb{N}_1, \leq)$ , model (3.2) is equivalent to the logistic regression model*

$$\text{pr}(Y = 1 \mid Z) = \frac{\exp(\gamma + \beta^\top Z)}{1 + \exp(\gamma + \beta^\top Z)} \text{ for some } \gamma \in \mathbb{R}. \quad (3.3)$$

Next, we show that in general, model (3.2) is implied by a logit model



## 3.2 Model-generating mechanisms

generalized from the continuation-ratio model for ordinal responses (Armstrong & Sloan, 1989), as proved in the Appendix.

**Proposition 3** (Sufficiency of generalized continuation-ratio model). *Model (3.2) is implied by the following generalized continuation-ratio model:*

$$\text{pr}(Y \succ y \mid Y \succeq y; Z) = \frac{\exp\{\gamma(y) + \beta^\top Z\}}{1 + \exp\{\gamma(y) + \beta^\top Z\}} \text{ for some } \gamma : \mathcal{Y} \rightarrow \mathbb{R}. \quad (3.4)$$

Under model (3.4), the conditional continuation ratio at  $y$  is  $\text{pr}(Y \succ y \mid Y \succeq y; Z) / \text{pr}(Y = y \mid Y \succeq y; Z) = \exp\{\gamma(y) + \beta^\top Z\}$ . This is the odds for a strictly “better” outcome given that it is comparable to and no worse than  $y$ . The model specifies that the covariate effects are invariant to the level  $y \in \mathcal{Y}$ , which imposes a strong constraint across outcome levels. In contrast, the multiplicative win ratio model specifies only the overall relationship between the response and covariates without level-specific constraints, making it more relaxed.

**Proposition 4** (Non-necessity of generalized continuation-ratio model).

*There exist multiplicative win ratio models that do not satisfy (3.4).*

*Proof.* Consider a counter-example with poset outcomes in  $(\mathbb{N}_{m-1}, \leq)$  ( $m > 2$ ) and a binary covariate  $Z \in \{1, 0\}$ . Model (3.2) then becomes a saturated model, which holds trivially with  $\beta = \log\{\mathcal{R}(\nu_1, \nu_0)\}$ , where  $(Y \mid Z = z) \sim \nu_z$  ( $z = 1, 0$ ). On the other hand, model (3.4) imposes a real constraint. To

### 3.3 Estimating equations

see this, write  $\lambda_{zk} = \text{pr}(Y > k \mid Y \geq k; Z = z)$ . With  $\text{logit}(x) = \log\{x/(1-x)\}$ , the model implies that  $\text{logit}(\lambda_{1k}) - \text{logit}(\lambda_{0k})$ , the log-continuation odds ratio at level  $k$ , is constant across  $k = 0, 1, \dots, m-2$ , which need not be true as  $(\lambda_{z0}, \dots, \lambda_{z,m-2})^T$  can vary freely in  $(0, 1)^{\otimes(m-1)}$ .

□

### 3.3 Estimating equations

Let  $(Y_i, Z_i)$  ( $i = 1, \dots, n$ ) be a random  $n$ -sample of  $(Y, Z)$ . To fit model (3.2), consider the pairwise error term

$$\mathcal{E}_{ij}(\beta) = I(Y_i \succ Y_j) \exp(\beta^T Z_j) - I(Y_j \succ Y_i) \exp(\beta^T Z_i), \quad i, j = 1, \dots, n.$$

Under (3.2), it is easy to see that  $E\{\mathcal{E}_{ij}(\beta) \mid Z_i, Z_j\} = 0$ . This motivates the covariate-weighted  $U$ -estimating function

$$\mathcal{U}_n(\beta; \hat{W}) = \binom{n}{2}^{-1} \sum_{i < j} \sum (Z_i - Z_j) \hat{W}(Z_i, Z_j; \beta) \mathcal{E}_{ij}(\beta), \quad (3.5)$$

where  $\hat{W}(\cdot, \cdot; \beta)$  is some data-dependent symmetric function on  $\mathcal{Z}^{\otimes 2}$  and  $\mathcal{Z} \subset \mathbb{R}^p$  is the covariate space. The estimator  $\hat{\beta}$  solves  $\mathcal{U}_n(\hat{\beta}; \hat{W}) = 0$ . Under suitable regularity conditions, finding  $\hat{\beta}$  can be reformulated as a minimization problem for a strictly convex function (see supplementary material for details), allowing us to use the standard Newton–Raphson algorithm. With a binary  $Z$ , it is easily seen that  $\hat{\beta}$  under every  $\hat{W}$  reduces to the log of the

### 3.3 Estimating equations

empirical two-sample win ratio of Section 2.2.

To estimate the variance of  $\hat{\beta}$ , the correlations between the terms in  $\mathcal{U}_n(\beta; \hat{W})$  must be accounted for. The following theorem, proved in the supplementary material, establishes the asymptotic properties of  $\hat{\beta}$  using uniform central limit theorems for  $U$ -processes (e.g., Arcones & Giné, 1993, Theorem 4.10) under the following regularity conditions. Let  $\beta_0$  denote the true value of  $\beta$ .

(C1) The covariate space  $\mathcal{Z}$  is bounded and the covariance matrix of  $Z$  is positive definite.

(C2) With  $(Y_i, Z_i) \perp\!\!\!\perp (Y_j, Z_j)$ , we have that  $\text{pr}(R_{ij} = 1 \mid Z_i, Z_j) > 0$  with probability one, where  $R_{ij} = I(Y_i \succ Y_j) + I(Y_j \succ Y_i)$ .

(C3) The weight function  $\hat{W}$  satisfies

$$\|\hat{W} - w\|_\infty \equiv \sup_{(z, z^*) \in \mathcal{Z}^{\otimes 2}, \beta \in \mathbb{R}^p} |\hat{W} - w|(z, z^*; \beta) \rightarrow_p 0, \quad (3.6)$$

for some limit function  $w(z, z^*; \beta)$  that is strictly positive, uniformly bounded, and continuous in  $\beta$  at  $\beta_0$ .

**Remark 2.** The boundedness of covariates in (C1) is imposed only to simplify the proof and may not be necessary in practice. As shown in the simulations in Section 4, unbounded distributions like the Gaussian

### 3.3 Estimating equations

distribution work well so long as their tail probabilities are not too heavy (see, e.g., Andersen & Gill, 1982). Condition (C2) can be viewed as non-degeneracy of the outcome distribution with respect to the partial order, and is satisfied if there are at least two comparable points in  $\mathcal{Y}$  with strictly positive conditional probabilities. Condition (C3) basically ensures that the weight function is asymptotically stable with a well-behaved limit.

**Theorem 2.** *Under conditions (C1)–(C3), we have that  $\hat{\beta} \rightarrow_p \beta_0$  and*

$$n^{1/2}(\hat{\beta} - \beta_0) = 2n^{-1/2}\Omega^{-1} \sum_{i=1}^n \psi(Y_i, Z_i) + o_p(1) \quad \text{as } n \rightarrow \infty, \quad (3.7)$$

where  $\Omega = E[R_{ij}(Z_i - Z_j)^{\otimes 2} w(Z_i, Z_j; \beta_0) \{\exp(-\beta_0^T Z_i) + \exp(-\beta_0^T Z_j)\}^{-1}]$

and

$$\psi(y, z) = E[(z - Z)w(z, Z; \beta_0) \{I(Y \prec y) \exp(\beta_0^T Z) - I(Y \succ y) \exp(\beta_0^T z)\}].$$

Estimators  $(\hat{\Omega}, \hat{\psi})$  of  $(\Omega, \psi)$  can be constructed by replacing the expectations with their empirical analogs,  $w$  with  $\hat{W}$ , and  $\beta_0$  with  $\hat{\beta}$ . Then the asymptotic variance of  $\hat{\beta}$  can be consistently estimated by the second moment of the influence function (Bickel et al., 1993) on the right hand side of (3.7), i.e.,  $\hat{\Sigma} = 4n^{-1}\hat{\Omega}^{-1} \{n^{-1} \sum_{i=1}^n \hat{\psi}(Y_i, Z_i)^{\otimes 2}\} \hat{\Omega}^{-1}$ . We can thus make inference on  $\beta_0$  based on the consistency and asymptotic normality of  $\hat{\beta}$  along with this variance estimator.

### 3.4 Efficiency consideration

It is clear from Theorem 2 that the asymptotic distribution of  $\hat{\beta}$  depends on  $\hat{W}(\cdot, \cdot; \beta)$  only through its limit  $w(\cdot, \cdot; \beta)$  at  $\beta = \beta_0$ . Consequently, if we substitute  $\beta$  in the weight function for some initial consistent estimator  $\hat{\beta}_{\text{init}}$ , the asymptotic property of the resulting estimator should remain the same. This substitution can save considerable computation when the target weight function  $\hat{W}(\cdot, \cdot; \beta)$  itself requires an iterative numerical procedure to compute for each  $\beta$ , such as  $\hat{W}_{\text{eff}}$  in (3.8) below. In such cases, solving the original equation  $\mathcal{U}_n(\beta; \hat{W}) = 0$  would necessitate an inner loop to recompute  $\hat{W}(\cdot, \cdot; \beta)$  at each iteration of the Newton–Raphson algorithm.

**Corollary 1.** *Let  $\hat{W}(\cdot, \cdot; \beta)$  be a weight function satisfying condition (C3) and let  $\hat{\beta}_{\text{init}}$  be an initial estimator such that  $\hat{\beta}_{\text{init}} \rightarrow_p \beta_0$ . Write  $\hat{W}_{\text{init}}(\cdot, \cdot) = \hat{W}(\cdot, \cdot; \hat{\beta}_{\text{init}})$ . Then, the estimators solving  $\mathcal{U}_n(\beta; \hat{W}) = 0$  and  $\mathcal{U}_n(\beta; \hat{W}_{\text{init}}) = 0$  are asymptotically equivalent.*

### 3.4 Efficiency consideration

The simplest choice for the weight is just  $\hat{W} \equiv 1$ , but this choice may not produce a statistically efficient  $\hat{\beta}$ . To improve efficiency, we exploit the equivalency between the multiplicative win ratio and logistic regression models in the case of a binary outcome (Proposition 2).

The basic strategy is outlined as follows. We first rewrite the effi-

### 3.4 Efficiency consideration

cient score of logistic regression in the pairwise form of (3.5), with weight  $\hat{W}(Z_i, Z_j; \beta) = \hat{W}_{\text{eff}}(Z_i, Z_j; \beta)$  for some  $\hat{W}_{\text{eff}}(Z_i, Z_j; \beta)$ . By standard likelihood theory,  $\hat{W}_{\text{eff}}(Z_i, Z_j; \beta)$  must be the efficient weight in the binary case. In the general case, because model (3.2) does not completely specify the likelihood, we construct pseudo-efficient weights by mimicking the form of  $\hat{W}_{\text{eff}}(Z_i, Z_j; \beta)$ . While the efficiency of pseudo-efficient weights is not theoretically guaranteed, we hope that they will at least improve upon naive weights like  $\hat{W} \equiv 1$ . As the first step, the following lemma exhibits the form of  $\hat{W}_{\text{eff}}(Z_i, Z_j; \beta)$ , with proof provided in the supplementary material.

**Lemma 2** (Efficient weight for binary data). *Under  $(\mathbb{N}_1, \leq)$ , let*

$$\hat{W}_{\text{eff}}(Z_i, Z_j; \beta) = [1 + \exp\{\hat{\gamma}(\beta) + \beta^T Z_i\}]^{-1} [1 + \exp\{\hat{\gamma}(\beta) + \beta^T Z_j\}]^{-1}, \quad (3.8)$$

where  $\hat{\gamma}(\beta)$  solves

$$\sum_{i=1}^n \left[ Y_i - \frac{\exp\{\hat{\gamma}(\beta) + \beta^T Z_i\}}{1 + \exp\{\hat{\gamma}(\beta) + \beta^T Z_i\}} \right] = 0. \quad (3.9)$$

Then, the  $U$ -estimating function  $\mathcal{U}_n(\beta; \hat{W}_{\text{eff}})$  reduces to a constant multiple of the efficient score function for  $\beta$  in the logistic regression model (3.3) and is hence efficient.

In the general setting, the form of  $\hat{W}_{\text{eff}}(Z_i, Z_j; \beta)$  in (3.8) applies without change. However, the definition of  $\hat{\gamma}(\beta)$  in (3.9) needs adaptation, as the

### 3.4 Efficiency consideration

outcome  $Y$  is no longer binary or numeric. We resolve this by mapping the partially ordered  $Y$  monotonically onto a numeric scale.

**Proposition 5** (Pseudo-efficient weights in general). *Let  $r : \mathcal{Y} \rightarrow [0, 1]$  be a strictly increasing scoring function in the sense that it maps the least and greatest elements in  $\mathcal{Y}$ , if existent, to 0 and 1, respectively, and that  $r(y_i) < r(y_j)$  for any  $y_i \prec y_j$ . Let  $\hat{\beta}_{\text{init}}$  denote a consistent initial estimator for  $\beta_0$ , e.g.,  $\hat{\beta}$  obtained under the naive weight  $\hat{W} \equiv 1$ . Define the pseudo-efficient weight by*

$$\hat{W}_{\text{pseff}}(Z_i, Z_j) = \left[ 1 + \exp\{\hat{\gamma}(\hat{\beta}_{\text{init}}) + \hat{\beta}_{\text{init}}^{\text{T}} Z_i\} \right]^{-1} \left[ 1 + \exp\{\hat{\gamma}(\hat{\beta}_{\text{init}}) + \hat{\beta}_{\text{init}}^{\text{T}} Z_j\} \right]^{-1}, \quad (3.10)$$

where  $\hat{\gamma}(\cdot)$  is defined as in (3.9) except with  $Y_i$  replaced by  $r(Y_i)$ . Then, the estimating function  $\mathcal{U}_n(\beta; \hat{W}_{\text{pseff}})$  leads to an efficient estimator for  $\beta$  under  $(\mathbb{N}_1, \leq)$ .

The proof of Proposition 5 is given in the Appendix. For  $(\prod_{k=1}^K \mathbb{N}_{m_k-1}, \leq)$  in Example 2, any averaging score of the form  $r(y) = K^{-1} \sum_{k=1}^K r_{ki_k}$  with  $y = (i_1, \dots, i_K)^{\text{T}}$  and  $0 = r_{k0} < r_{k1} < \dots < r_{k, m_k-1} = 1$  satisfies the requirements for a strictly increasing scoring function. Even in the general case, it is usually straightforward to construct a proper scoring function by following through a Hasse diagram (Trotter, 1992) for the partial order. An

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example is offered in the supplementary material for the non-lattice poset from the smoking behavior study described in Section 2.1 (Zhang & Ip, 2012).

Both the naive and pseudo-efficient analyses of win ratio regression models are implemented in the R-package `poset`, which is publicly available on GitHub at <https://lmaowisc.github.io/poset> and on the Comprehensive R Archive Network (CRAN).

#### 4. Simulation studies

We first assessed the empirical power of the win ratio test under the proportional odds model for ordinal data in comparison with standard tests. We considered two scenarios, one under  $(\mathbb{N}_2, \leq)$  with  $\nu_0(0) = \nu_0(1) = 0.3$  and  $\nu_0(2) = 0.4$ , and the other under  $(\mathbb{N}_4, \leq)$  with  $\nu_0(0) = \nu_0(3) = 0.1$ ,  $\nu_0(1) = 0.2$ , and  $\nu_0(2) = \nu_0(4) = 0.3$ . We compared the win ratio with three other tests:

1. The parametric Wald test based on the maximum-likelihood log-odds ratio estimator under the proportional odds model.
2. The chi-square test on the nominal levels.
3. The binomial test on the responses dichotomized by  $\{0\}$  vs  $\{1, 2\}$  for



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$(\mathbb{N}_2, \leq)$  and  $\{0, 1\}$  vs  $\{2, 3, 4\}$  for  $(\mathbb{N}_4, \leq)$ .

Under varying odds ratios, we computed and plotted the empirical powers of the four tests for  $n = 200$  in Figure 1. In both scenarios, the asymptotic power function for the efficient test given in the supplementary material provides accurate approximation to the empirical powers of both the win ratio and parametric tests, which outperform those of the other two tests by considerable margins. These results confirm the asymptotic efficiency of the win ratio under proportional odds alternatives, as stated in Theorem 1 (b).

Next, we assessed the inference procedures described in Sections 3.3 and 3.4 for the multiplicative regression model. Set  $(\mathcal{Y}, \preceq) = (\mathbb{N}_2 \times \mathbb{N}_2, \leq)$  and  $Z = (Z_1, Z_2)^\top$ , where  $Z_1 \sim N(0, 1)$  and  $Z_2 = 2 \times \text{Bernoulli}(0.5) - 1$ . To generate  $Y$  given  $Z$ , we used the generalized continuation-ratio logit model in (3.4). It can be shown, with details in the supplementary material, that model (3.4) in this case completely determines the conditional distribution of the outcome. Set  $\gamma_{00} = 2.0$ ,  $\gamma_{01} = \gamma_{10} = 1.0$ ,  $\gamma_{02} = \gamma_{20} = \gamma_{11} = 0.2$ , and  $\gamma_{21} = \gamma_{12} = 0.1$ , where  $\gamma_y$  is a shorthand notation for  $\gamma(y)$ . Under this set-up, the conditional probabilities for the nine levels of the outcome given  $Z = (0, 0)^\top$  are roughly bounded between 0.05 and 0.20. For  $n = 200, 500, 1000$  and  $\beta = (\beta_1, \beta_2)^\top = (0, 0)^\top, (0.25, -0.25)^\top, (0.5, -0.5)^\top$ , we

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assessed the estimation of  $\beta_1$  using both the naive estimator (derived from  $\mathcal{U}_n(\beta; \hat{W})$  with  $\hat{W} \equiv 1$ ) and the pseudo-efficient estimator. The latter was constructed using the naive estimator as  $\hat{\beta}_{\text{init}}$  to form the pseudo-efficient weight in (3.10) with the average scoring function  $r\{(i_1, i_2)^\top\} = 4^{-1}(i_1 + i_2)$ .

The results for both estimators are summarized in Table 1. Both exhibit minimal bias, with largely accurate standard error estimators and confidence intervals. Under  $\beta = (0, 0)^\top$ , the relative efficiency between the two estimators is close to one. This is unsurprising, as the pseudo-efficient weight in (3.10) is asymptotically constant in this case, making it equivalent to the naive weight. As the magnitude of  $\beta$  increases, the pseudo-efficient estimator becomes more efficient, with efficiency gains of over 30% under  $\beta = (0.5, -0.5)^\top$  compared to the naive estimator. The efficiency difference is expected to widen under stronger covariate-response associations. Interestingly, the pseudo-efficient estimator and the associated variance estimator also appear to be more accurate than the naive versions for smaller sample sizes.

Additional simulations in the supplementary material explore other scoring functions for the pseudo-efficient weight, including transformations that produce skewed scores (e.g., square or square-root transformations). The resulting pseudo-efficient estimators perform similarly and all outper-

Table 1: Simulation results for estimation of  $\beta_1$  in the multiplicative win ratio model.

$n$	$\beta_1$	Naive				Pseudo-efficient				
		Bias	SE	SEE	CP	Bias	SE	SEE	CP	RE
200	0	0.000	0.117	0.115	0.945	0.000	0.114	0.115	0.951	1.05
	0.25	0.008	0.123	0.121	0.948	0.001	0.115	0.118	0.956	1.14
	0.50	0.016	0.147	0.137	0.932	-0.002	0.125	0.126	0.952	1.39
500	0	0.000	0.072	0.072	0.948	0.000	0.071	0.072	0.950	1.02
	0.25	0.001	0.077	0.076	0.950	-0.002	0.073	0.073	0.952	1.11
	0.50	0.008	0.090	0.088	0.940	0.000	0.078	0.078	0.953	1.33
1000	0	0.000	0.050	0.050	0.953	0.000	0.050	0.050	0.952	1.01
	0.25	0.001	0.054	0.054	0.951	0.000	0.051	0.051	0.953	1.12
	0.50	0.004	0.064	0.063	0.945	0.000	0.055	0.055	0.946	1.35

SE, empirical standard error of the estimator; SEE, empirical average of the standard error estimator; CP, empirical coverage rate of the 95% confidence interval. RE, relative efficiency, i.e., inverse ratio of the empirical variance, comparing the pseudo-efficient versus naive estimators. Each entry is based on 5,000 replicates.

form the naive approach. A case with ordinal outcomes is also considered in the supplementary material.

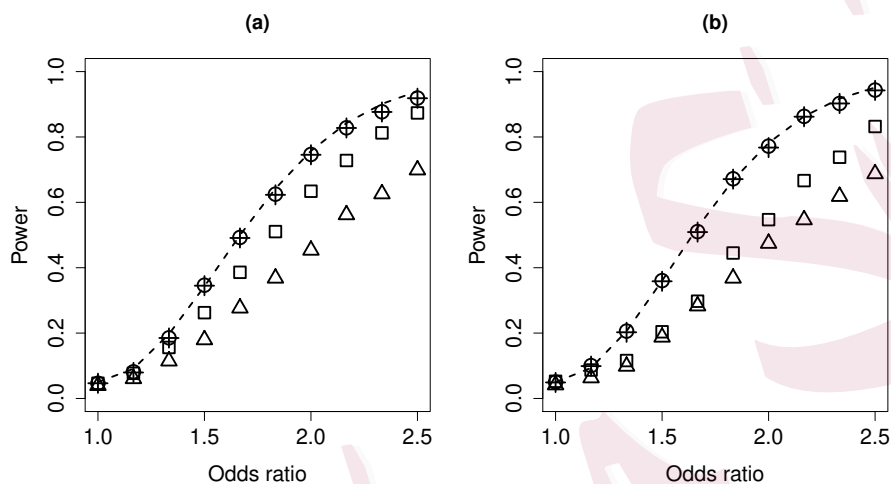


Figure 1: Empirical power as a function of the odds ratio under the proportional odds model for (a) three-level and (b) five-level ordinal outcomes with  $n = 200$ . Dashed line: asymptotic power for the efficient test; plus sign: empirical power of the win ratio test; circle: parametric Wald test; square: chi-square test on nominal levels; triangle: binomial test on dichotomized outcome. Each value of the empirical power is based on 2,000 replicates.

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## 5. Real examples

### 5.1 A radiologic study of liver disease

A total of 186 patients with non-alcoholic fatty liver disease (NAFLD) were recruited at the University of Wisconsin Hospitals in 2017. The patients underwent computed tomography scan of the liver for the presence of non-alcoholic steato-hepatitis (NASH), the most severe form of NAFLD. The images were subsequently assessed by two radiologists using a scale of 1 to 5, with higher values indicating a greater likelihood of disease. Descriptive statistics on the study cohort are tabulated in the supplementary material.

For win ratio analysis of the reader assessments, we invert the scores so that higher values indicate a lower likelihood of disease and are thus more favorable. Because the two readers have similar levels of experience, we apply the product order to their bivariate scores. This results in an outcome space represented by  $\mathcal{Y} = \mathbb{N}_4 \times \mathbb{N}_4$ . Under the product order, the win ratio measures the fold change in the probability of achieving a favorable rating by consensus of the two readers.

We employ the multiplicative win ratio model of Section 3 to examine the relationship between the radiologists' scores and several covariates: patient sex, the presence of advanced fibrosis (AF), and quantitative biomark-

## 5.1 A radiologic study of liver disease

Table 2: Win ratio regression analysis of the non-alcoholic fatty liver disease study data.

	Naive			Pseudo-efficient		
	Estimate	Std error	$p$ -value	Estimate	Std error	$p$ -value
Sex (f v. m)	-0.144	0.270	0.593	-0.151	0.262	0.563
AF (y v. n)	-0.890	0.307	0.004	-0.903	0.296	0.002
Steatosis (%)	-0.026	0.006	<0.001	-0.027	0.005	<0.001
Gray level	-0.010	0.006	0.070	-0.010	0.005	0.065
LSN score	-0.062	0.134	0.646	-0.074	0.130	0.571

ers such as percent of steatosis (liver fat content), liver mean gray level intensity, and liver surface nodularity (LSN) score. Like in the simulations, we first fit the model using the naive procedure, followed by the pseudo-efficient one utilizing an average scoring function similar to that in Section 4. The results are summarized in Table 2. While the point estimates of the regression coefficients are comparable between the two methods, the pseudo-efficient estimators consistently exhibit smaller standard errors and lower  $p$ -values, indicating enhanced efficiency over the naive estimators.

By the recommended pseudo-efficient approach, advanced fibrosis status and percent of steatosis are strongly and significantly associated with the likelihood of NASH. In particular, patients with advanced fibrosis are

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## 5.2 The youth tobacco use study

$\exp(-0.89) = 41.1\%$  times as likely to receive favorable assessments than those without. Additionally, one percent increase in steatosis results in  $1 - \exp(-0.026) = 2.6\%$  reduction in the likelihood of favorable assessments.

For comparison, we use the continuation-ratio model to analyze the sum of the two radiologists' scores against the same set of covariates. As detailed in the supplementary material, the regression coefficients obtained are comparable to those in Table 2. Notably, the effects of advanced fibrosis and steatosis remain highly significant, while the association with mean gray level intensity becomes less pronounced. These findings suggest that, despite the fewer assumptions inherent in the win ratio regression, it maintains a level of efficiency comparable to that of parametric methods.

### 5.2 The youth tobacco use study

The youth tobacco use study mentioned in Section 2.1 was analyzed using the partitioned conditional model (PCM) (Zhang & Ip, 2012). The outcome was the subject's smoking status classified into six partially ordered levels ( $y = 0, 1, \dots, 5$ ), as illustrated in the Hasse diagram in Figure 2. Nonsmoker was considered the most desirable status, while heavy frequent smoker was the least desirable. The PCM consisted of three or-

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## 5.2 The youth tobacco use study

dinal/nominal sub-models, generating three sets of regression coefficients. Many of these coefficients, such as the log-odds between light frequent vs. heavily infrequent smoking, may not be of direct interest if the primary goal is to assess risk factors for undesirable behavior.

As a comparison, we apply the win ratio approach to a mock dataset of the study (Zhang & Ip, 2012). The data consist of 3370 male and 5411 female youths aged 12 to 16 years. Predictors of smoking behavior include sex, race, whether living with parents, having a nonsupportive (NS) mother, having strict parents, attending school, having a negative attitude towards discipline, and having smoking peers. Descriptive statistics of these variables, as well as the outcome, are summarized in Table 3.

We use the multiplicative win ratio model to analyze the effects of these predictors on the smoking status. We first fit the model using the naive weight. Then, with the monotonic scoring function  $r(0) = 0, r(1) = r(2) = 1/3, r(3) = r(4) = 2/3$ , and  $r(5) = 1$ , we compute the pseudo-efficient estimators. The results are summarized in Table 4. Not surprisingly, the pseudo-efficient estimates are similar to the naive ones in magnitude, but with generally smaller standard errors. Except for age, all predictors are highly significantly associated with smoking behavior. In particular, male youths are  $1 - \exp(-1.087) = 66.3\%$  less likely to have a desirable smoking



## 5.2 The youth tobacco use study

Table 3: Descriptive statistics for the National Longitudinal Study of Youth on tobacco use.

		Female	Male	Overall
Age	(years)	14.0 (13.1, 14.9)	13.9 (13.0, 15.0)	14.0 (13.0,15.0)
Nonwhite		954 (28.3%)	1174 (21.7%)	2128 (24.2%)
Live with parents		1119 (33.2%)	1601 (29.6%)	2720 (31%)
NS mother		2305 (68.4%)	4061 (75.1%)	6366 (72.5%)
Strict parents		1032 (30.6%)	1268 (23.4%)	2300 (26.2%)
Attend school		2704 (80.2%)	4823 (89.1%)	7527 (85.7%)
Neg. discipline		2398 (71.2%)	4218 (78%)	6616 (75.3%)
Smoking peers		2609 (77.4%)	4647 (85.9%)	7256 (82.6%)
Smoking status	0	141 (2.6%)	658 (19.5%)	799 (9.1%)
	1	58 (1.1%)	147 (4.4%)	205 (2.3%)
	2	36 (0.7%)	52 (1.5%)	88 (1%)
	3	955 (17.6%)	923 (27.4%)	1878 (21.4%)
	4	334 (6.2%)	168 (5%)	502 (5.7%)
	5	3887 (71.8%)	1422 (42.2%)	5309 (60.5%)

Categorical variables are summarized by  $N(\%)$  and quantitative variables by median (inter-quartile range).

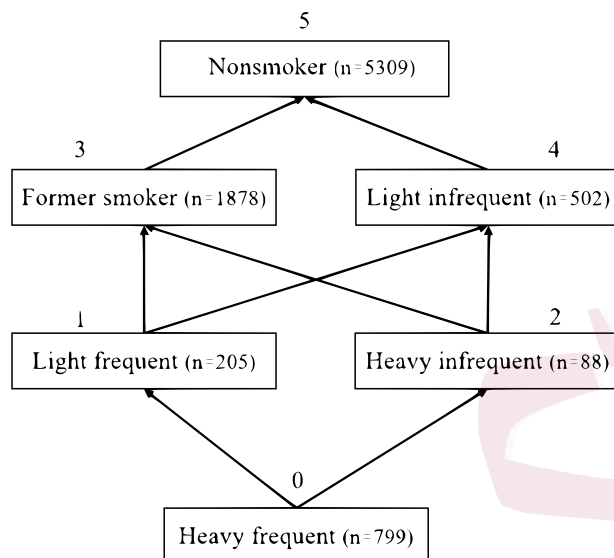


Figure 2: Hasse diagram from Zhang & Ip (2012) for the National Longitudinal Study of Youth on tobacco use.

behavior than female youths. As sensitivity analysis, an alternative scoring function is considered in the supplementary material, which shows similar results to the original pseudo-efficient analysis.

## 6. Concluding remarks

This win ratio framework can be immediately extended to a stratified analysis, which is desirable if there is considerable between-strata heterogeneity. Let  $X \in \mathcal{X}$  denote the categorical variable to be stratified on, e.g., sex, race, or age groups, where  $\mathcal{X}$  is a discrete space. To restrict comparisons within

Table 4: Win ratio regression analysis of smoking behavior in the National Longitudinal Study of Youth on tobacco use.

	Naive			Pseudo-efficient		
	Estimate	Std error	<i>p</i> -value	Estimate	Std error	<i>p</i> -value
Male	-1.087	0.046	<0.001	-1.156	0.043	<0.001
Nonwhite	0.623	0.053	<0.001	0.681	0.047	<0.001
Age (years)	0.014	0.021	0.514	0.025	0.018	0.155
Live w. parents	0.442	0.051	<0.001	0.508	0.044	<0.001
NS mother	-0.537	0.052	<0.001	-0.602	0.045	<0.001
Strict parents	0.547	0.052	<0.001	0.623	0.045	<0.001
Attend school	1.175	0.062	<0.001	1.221	0.056	<0.001
Neg. discipline	-0.655	0.053	<0.001	-0.722	0.046	<0.001

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each stratum, simply treat  $\mathcal{Y} \times \mathcal{X}$  as the new outcome space, equipped with the partial order  $\preceq_s$  such that  $(y_i, x_i) \preceq_s (y_j, x_j)$  if and only if  $y_i \preceq y_j$  and  $x_i = x_j$ . Under this formulation, the results for nonparametric inference and semiparametric regression carry over without change.

Compared to standard parametric models, the win ratio provides a parsimonious approach to treatment or covariate effects, by focusing only on the global favorability of outcomes under the partial order. Nevertheless, there may be situations where parametric methods are preferred, particularly if the model suits the context of application. For example, the continuation-ratio model (3.4) allows us to estimate the odds of moving to a higher category given the current status, providing intuitive interpretations for outcomes that proceed in stages, like educational attainment (Agresti, 2010). A smaller sample size may also favor parametric methods (pending further investigation). On the other hand, an elaborate model increases the risk of misspecification, and residual analysis should be used to check model fit whenever possible.

The efficient analysis of partially ordered data has received insufficient attention in the literature, and Theorem 1 and Proposition 5 provide only preliminary results on this topic. For the multiplicative win ratio model, in particular, the pseudo-efficient estimators show improvements over the

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naive ones but are not guaranteed to be globally optimal. A complete semiparametric efficiency theory, traditionally relying on the concept of influence functions (Bickel et al., 1993), is complicated by the pairwise formulation of the model, which makes it hard to isolate the influence of each individual. Recently, Vermeulen et al. (2023) examined the efficiency problem for the probabilistic index model (Thas et al., 2012), a pairwise-defined model for continuous and ordinal outcomes. Their strategies may help shed light on the structurally similar win ratio regression.

## Appendix

### Proof of Lemma 1

The inequalities follow by Definition 1 and every  $U(y)$  being an up-set. For strictness, suppose for a contradiction that  $\nu_1\{U(y)\} = \nu_0\{U(y)\}$  for all  $y$ . Since the  $U[y]$  are also up-sets, we must have that  $\nu_1(U[y]) \geq \nu_0(U[y])$ .

This means that  $\nu_1(y) \geq \nu_0(y)$  for all  $y \in \mathcal{Y}$ , which is impossible unless  $\nu_1(y) = \nu_0(y)$  for all  $y \in \mathcal{Y}$ , contradicting the assumption that  $\nu_1 \succ \nu_0$ .

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## Proof of Proposition 2

For sufficiency of (3.3), use calculations similar to the proof of Proposition 1 to find that

$$\begin{aligned} \mathcal{R}(Z_i, Z_j) &= \frac{\text{pr}(Y_i = 1 \mid Z_i)\text{pr}(Y_j = 0 \mid Z_j)}{\text{pr}(Y_i = 0 \mid Z_i)\text{pr}(Y_j = 1 \mid Z_j)} = \frac{\exp(\gamma + \beta^\top Z_i)}{\exp(\gamma + \beta^\top Z_j)} \\ &= \exp\{\beta^\top(Z_i - Z_j)\}, \end{aligned} \quad (6.11)$$

where the second equality follows by  $\text{pr}(Y = 1 \mid Z)/\text{pr}(Y = 0 \mid Z) = \exp(\gamma + \beta^\top Z)$  under model (3.3). For necessity, take  $Z_i = Z$  and  $Z_j = z_0$  for some fixed  $z_0$  in (3.2) and use the first equality in (6.11) to obtain (3.3) with  $\gamma = \log\{\text{pr}(Y = 1 \mid z_0)/\text{pr}(Y = 0 \mid z_0)\} - \beta^\top z_0$ .

## Proof of Proposition 3

Let  $S(y \mid Z) = \text{pr}(Y \succeq y \mid Z)$ . Under model (3.4), the numerator of  $\mathcal{R}(Z_i, Z_j)$  is

$$\begin{aligned} &\text{pr}(Y_i \succ Y_j \mid Z_i, Z_j) \\ &= \sum_{y \in \mathcal{Y}} \text{pr}(Y_j = y \mid Z_j)\text{pr}(Y_i \succ y \mid Z_i) \\ &= \sum_{y \in \mathcal{Y}} \text{pr}(Y_i \succ y \mid Y_i \succeq y; Z_i)\text{pr}(Y_j = y \mid Y_j \succeq y; Z_j)S(y \mid Z_i)S(y \mid Z_j) \\ &= \exp(\beta^\top Z_i) \sum_{y \in \mathcal{Y}} \frac{\exp\{\gamma(y)\}S(y \mid Z_i)S(y \mid Z_j)}{[1 + \exp\{\gamma(y) + \beta^\top Z_i\}][1 + \exp\{\gamma(y) + \beta^\top Z_j\}]}. \end{aligned} \quad (6.12)$$

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By symmetry, the denominator of  $\mathcal{R}(Z_i, Z_j)$  is the far right hand side of (6.12) with the factor  $\exp(\beta^\top Z_i)$  replaced with  $\exp(\beta^\top Z_j)$ . This yields the desired form of  $\mathcal{R}(Z_i, Z_j)$  in (3.2).

### **Proof of Propostion 5**

Any qualified scoring function  $r$  reduces to the identity function under  $(\mathbb{N}_1, \leq)$ . By Lemma (2), the weight defined by the right hand side of (3.10) with  $\hat{\beta}_{\text{init}}$  replaced by  $\beta$  is efficient. But by Corollary 1, the replacement of  $\beta$  by  $\hat{\beta}_{\text{init}}$  does not alter the asymptotic distribution of the resulting estimator.

### **Supplementary Materials**

Supplementary materials include technical results and additional numerical studies. An R-package `poset` that implements the proposed methodology is available on GitHub at <https://lmaowisc.github.io/poset> as well as the Comprehensive R Archive Network (CRAN), both with a tutorial based on the liver study in Section 5.1.

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