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TITLE: NOVEL EMPIRICAL LIKELIHOOD METHODS FOR TWO-SAMPLE  
PROBLEMS AND THE APPLICATIONS IN SURVIVAL ANALYSIS

by

KANGNI ALEMDJRODO

Under the Direction Yichuan Zhao, PhD

ABSTRACT

Empirical likelihood (EL) is a nonparametric method inspired by the usual maximum likelihood. There has been a wide range of applications to different statistical parameters since Owen's works (1988, 1990). While EL has many advantages over existing inference methods, it also has some flaws: heavy computations, low accuracy for the small samples and high-dimensional applications, etc. In this dissertation, we investigate some EL's extensions, namely the jackknife empirical likelihood (JEL), the i.i.d. empirical likelihood (IID

EL), and the weighted empirical likelihood (WEL) in constructing confidence intervals (CI) for particular parameters of interest. It contributes to significantly improving the CI by substantially reducing the extensive computation associated with the EL method, ameliorating the poor performance of EL for the small sample and heavy-tailed distributions.

We propose a new plug-in approach of JEL to reduce the computational cost in comparing two Gini indices for paired data. One of the main results of the EL is the nonparametric extension of Wilks' theorem for parametric likelihood ratios. However, this result is violated when the data is censored. To circumvent this issue for some specific parameters, we combine the EL method with the influence functions (IID EL) to construct a confidence interval for the mean residual life (MRL) function in the presence of length-bias. Further, we extend the IID EL to the two-sample mean difference, where the two samples considered are right-censored. Last, we consider the weighted empirical likelihood (WEL) for comparing the areas under two correlated ROC curves (AUC).

For the first three essays, we proved that Wilks' theorem holds: the log-likelihood ratio statistic is asymptotically chi-square distributed. And the WEL statistic has a scaled chi-squared distribution. The extensive simulations demonstrated that, for finite samples, all the proposed methods outperform the existing EL methods in coverage probability accuracy and average lengths of CI. Finally, the application to real data demonstrated that the proposed methods are of practical value.

INDEX WORDS: Area under the ROC curve, Gini index, I.i.d. empirical likelihood, Jackknife empirical likelihood, Length-bias, Mean difference, Mean residual life, Right censoring, Weighted empirical likelihood, Wilks' theorem.

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A Dissertation Submitted in Partial Fulfillment of the Requirements for the Degree of

Doctor of Philosophy  
in the College of Arts and Sciences  
Georgia State University

2020

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2020

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## DEDICATION

This dissertation is dedicated to my wife Akoko, my kids Shana, Sherlock, Sherman, Sheldon, my mum Françoise and my brother Eric, both posthumously.

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## TABLE OF CONTENTS

<b>ACKNOWLEDGEMENTS</b> . . . . .	<b>v</b>
<b>LIST OF TABLES</b> . . . . .	<b>x</b>
<b>LIST OF FIGURES</b> . . . . .	<b>xiii</b>
<b>LIST OF ABBREVIATIONS</b> . . . . .	<b>xiv</b>
<b>CHAPTER 1 INTRODUCTION</b> . . . . .	<b>1</b>
1.1 Empirical Likelihood . . . . .	1
1.2 Survival Data . . . . .	3
1.3 Summary . . . . .	4
<b>CHAPTER 2 REDUCE THE COMPUTATION IN JACKKNIFE EM- PIRICAL LIKELIHOOD FOR COMPARING TWO COR- RELATED GINI INDICES</b> . . . . .	<b>7</b>
2.1 Background . . . . .	7
2.2 Main Results . . . . .	9
2.2.1 Jackknife empirical likelihood (JEL) . . . . .	9
2.2.2 Adjusted jackknife empirical likelihood (AJEL) . . . . .	14
2.2.3 Bootstrap calibration . . . . .	15
2.3 Simulation Study . . . . .	16
2.4 Real Applications . . . . .	20
2.5 Conclusions . . . . .	23
<b>CHAPTER 3 NEW EMPIRICAL LIKELIHOOD INFERENCE FOR THE MEAN RESIDUAL LIFE WITH LENGTH-BIASED AND</b>	

<b>RIGHT-CENSORED DATA</b> . . . . .	<b>25</b>
<b>3.1 Background</b> . . . . .	25
<b>3.2 Main Results</b> . . . . .	28
3.2.1 New empirical likelihood (NEL) . . . . .	28
3.2.2 Adjusted new empirical likelihood (ANEL) . . . . .	32
<b>3.3 Simulation Study</b> . . . . .	33
<b>3.4 Real Applications</b> . . . . .	41
<b>3.5 Conclusions</b> . . . . .	43
<b>CHAPTER 4 NOVEL EMPIRICAL LIKELIHOOD INFERENCE FOR THE MEAN DIFFERENCE WITH RIGHT-CENSORED DATA</b> . . . . .	<b>44</b>
<b>4.1 Background</b> . . . . .	44
<b>4.2 Main Results</b> . . . . .	46
4.2.1 I.i.d. empirical likelihood (IID-EL) . . . . .	46
4.2.2 I.i.d. adjusted empirical likelihood (IID-AEL) . . . . .	52
4.2.3 I.i.d. mean empirical likelihood (IID-MEL) . . . . .	54
<b>4.3 Simulation Study</b> . . . . .	56
<b>4.4 Real Application</b> . . . . .	64
<b>4.5 Conclusions</b> . . . . .	65
<b>CHAPTER 5 WEIGHTED EMPIRICAL LIKELIHOOD INFERENCE FOR THE DIFFERENCE BETWEEN THE AREAS UN- DER TWO CORRELATED ROC CURVES WITH RIGHT- CENSORED DATA</b> . . . . .	<b>66</b>
<b>5.1 Background</b> . . . . .	66
<b>5.2 Main Results</b> . . . . .	69
5.2.1 Normal approximation (NA) . . . . .	71
5.2.2 Weighted empirical likelihood (WEL) . . . . .	72

5.2.3 Adjusted weighted empirical likelihood (AWEL) . . . . .	74
<b>5.3 Simulation Study . . . . .</b>	<b>76</b>
<b>5.4 Real Applications . . . . .</b>	<b>80</b>
<b>5.5 Conclusions . . . . .</b>	<b>81</b>
<b>CHAPTER 6 DISCUSSIONS AND FUTURE WORKS . . . . .</b>	<b>83</b>
<b>REFERENCES . . . . .</b>	<b>85</b>
<b>APPENDICES . . . . .</b>	<b>99</b>
<b>Appendix A PROOFS OF CHAPTER 2 . . . . .</b>	<b>99</b>
<b>Appendix B PROOFS OF CHAPTER 3 . . . . .</b>	<b>106</b>
<b>Appendix C PROOFS OF CHAPTER 4 . . . . .</b>	<b>112</b>
<b>Appendix D PROOFS OF CHAPTER 5 . . . . .</b>	<b>121</b>

## LIST OF TABLES

Table 2.1	Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods; $X, Y \sim U(0, 1)$ . . . . .	17
Table 2.2	Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods; $X, Y \sim exp(2)$ . . . . .	17
Table 2.3	Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods; $X, Y \sim Weibull(0.5, 2)$ . . . . .	18
Table 2.4	Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods; $X, Y \sim Lognormal(0, 1.5)$ . . . . .	18
Table 2.5	Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods; $X \sim Gamma(7.7, 1), Y \sim Uniform(1, 1.9)$ . . . . .	18
Table 2.6	Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods; $X \sim Lognormal(0, 1), Y \sim Pareto(1.5, 3)$ . . . . .	19
Table 2.7	Comparison of computing times (in seconds) for different empirical likelihood methods; $X, Y \sim U(0, 1)$ . . . . .	19
Table 2.8	Comparison of computing times (in seconds) for different empirical likelihood methods; $X, Y \sim exp(2)$ . . . . .	19
Table 2.9	Point and interval estimates for the parameters of interest from the PWT data set. . . . .	21

Table 2.10	Point and interval estimates for the parameters of interest from the PSID data set. . . . .	24
Table 3.1	Comparison of coverage probabilities (average lengths) of the confidence intervals with 10% censoring rate, for S1. . . . .	35
Table 3.2	Comparison of coverage probabilities (average lengths) of the confidence intervals with 30% censoring rate, for S1. . . . .	36
Table 3.3	Comparison of coverage probabilities (average lengths) of the confidence intervals with 10% censoring rate, for S2. . . . .	37
Table 3.4	Comparison of coverage probabilities (average lengths) of the confidence intervals with 30% censoring rate, for S2. . . . .	38
Table 3.5	Comparison of coverage probabilities (average lengths) of the confidence intervals with 10% censoring rate, for S3. . . . .	39
Table 3.6	Comparison of coverage probabilities (average lengths) of the confidence intervals with 30% censoring rate, for S3. . . . .	40
Table 3.7	Comparison of coverage probabilities (average length) of the 95% confidence intervals with 10% censoring rate, for S1. . . . .	40
Table 3.8	90% confidence intervals for the MRL at the selected ages for the Channing house data. . . . .	42
Table 3.9	90% confidence intervals for the MRL at the selected ages for men versus women for the Channing house data. . . . .	42
Table 4.1	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\theta$ at different censoring rates (CR) with 90% confidence level; $X, Y \sim \mathcal{U}(0, 1)$ and $U, V \sim \mathcal{U}(0, c_0)$ . . . . .	58
Table 4.2	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\theta$ at different censoring rates (CR) with 90% confidence level; $X \sim Exp(2), Y \sim ShiftedExp(2, 1), U \sim Exp(\lambda_1)$ and $V \sim Exp(\lambda_2)$ . . . . .	59

Table 4.3	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\theta$ at different censoring rates (CR) with 90% confidence level; $X \sim \text{logNorm}(0, 1), Y \sim \chi_3^2, U \sim \mathcal{U}(c_1, 2c_1)$ and $V \sim \text{Exp}(\lambda_3)$ . . . . .	60
Table 4.4	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\theta$ at different censoring rates (CR) with 95% confidence level; $X, Y \sim \mathcal{U}(0, 1)$ and $U, V \sim \mathcal{U}(0, c_0)$ . . . . .	61
Table 4.5	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\theta$ at different censoring rates (CR) with 95% confidence level; $X \sim \text{Exp}(2), Y \sim \text{ShiftedExp}(2, 1), U \sim \text{Exp}(\lambda_1)$ and $V \sim \text{Exp}(\lambda_2)$ . . . . .	62
Table 4.6	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\theta$ at different censoring rates (CR) with 95% confidence level; $X \sim \text{logNorm}(0, 1), Y \sim \chi_3^2, U \sim \mathcal{U}(c_1, 2c_1)$ and $V \sim \text{Exp}(\lambda_3)$ . . . . .	63
Table 4.7	90% and 95% Confidence intervals for $\theta$ for the PBC data . . . . .	65
Table 5.1	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\Delta = -0.1$ at different censoring rates (CR) with 95% confidence level. . . . .	77
Table 5.2	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\Delta = 0.05$ at different censoring rates (CR) with 95% confidence level. . . . .	78
Table 5.3	Comparison of coverage probabilities (average lengths) of the confidence intervals of $\Delta = 0.3$ at different censoring rates (CR) with 95% confidence level. . . . .	79
Table 5.4	95% Confidence intervals for $\Delta$ for the PBC data . . . . .	81

**LIST OF FIGURES**

Figure 1.1	Different types of censoring. . . . .	4
Figure 2.1	The Gini Index . . . . .	10
Figure 2.2	Exploratory plots for the PWT data set. . . . .	22
Figure 2.3	Exploratory plots for the PSID data set. . . . .	23
Figure 5.1	Comparison of AUCs for the PBC data set. . . . .	82



## LIST OF ABBREVIATIONS

- AEL - Adjusted empirical likelihood
- AJEL - Adjusted jackknife empirical likelihood
- ANEL - Adjusted new empirical likelihood
- AL - Average length
- AUC - Areas under the ROC curve
- AWEL - Adjusted weighted empirical likelihood
- CDF - Cumulative distribution function
- CI - Confidence Interval
- CP - Coverage probability
- ECDF - Empirical cumulative distribution function
- EL - Empirical likelihood
- ELR - Empirical likelihood Ratio
- EL-WW - Empirical likelihood Wang and Wang
- FNR - False negative rate
- FPR - False positive rate
- GDP - Gross domestic product
- GSU - Georgia State University
- i.i.d. - Independent and identically distributed

- IID-AEL - I.i.d. adjusted empirical likelihood
- IID-EL - I.i.d. empirical likelihood
- IID-MEL - I.i.d. mean empirical likelihood
- JEL - Jackknife empirical likelihood
- KM - Kaplan-Meier
- MEL - Mean empirical likelihood
- MEL - Maximum empirical likelihood estimator
- MRL - Mean residual life function
- NA - Normal approximation
- NEL - New empirical likelihood
- NPMLE - Nonparametric maximum likelihood estimator
- PSID - Panel Study of Income Dynamics
- PWT - Penn World Tables
- PBC - Primary Biliary Cirrhosis
- ROC - Receiver operating characteristic
- TNR - True negative rate
- TPR - True positive rate
- WEL - Weighted empirical likelihood

## CHAPTER 1

### INTRODUCTION

The introductory chapter will review the main concepts of empirical likelihood (EL) and its most essential properties, and will give a brief description of survival data. Then, we will present the results obtained during this dissertation.

#### 1.1 Empirical Likelihood

The EL can be seen as a nonparametric maximum likelihood, in which the empirical cumulative distribution function (ECDF) has replaced the unknown distribution function. Thomas and Grunkemeier (1975) introduced the EL to estimate survival functions with censored data. Owen (1988, 1990, 2001) generalized the method to complete data, a wide range of statistical functionals and many different statistical problems. As one of the best nonparametric methods used to derive confidence intervals, the empirical likelihood has many advantages: it is distribution-free, has weak regularity conditions, its confidence regions are Bartlett correctable, and the observed data determine their shapes, etc. Its advantage over the normal approximation (NA) method is no longer questionable (Hall and La Scala, 1990). There is no need to estimate the variance when constructing EL confidence intervals contrary to NA confidence intervals. The most attractive property of the EL method is it retains the Wilks' result (asymptotic theorem for the classic likelihood ratio tests) that is **-2log(EL ratio)** is asymptotically  $\chi^2$ -distributed (Owen, 1988).

Now we review the basic idea of EL as follows. Let  $\mathbf{X} = \{X_1, X_2, \dots, X_n\}$  be an independent and identically distributed (i.i.d.) random variable from an unknown distribution  $F$ . Suppose we observe  $X_i = x_i, i = 1, 2, \dots, n$ . Let  $p_i$  be a probability mass assigned to  $x_i$  :

$$p_i = P(X_i = x_i) = F(x_i) - F(x_i-)$$

with

$$p_i \geq 0, \quad \sum_{i=1}^n p_i = 1.$$

The EL function is  $L(F) = L(p_1, p_2, \dots, p_n; \mathbf{X}) = \prod_{i=1}^n p_i$ .  $L(F)$  is maximized at  $\hat{p}_i = 1/n$  and the likelihood  $L(F)$  reaches its maximum  $n^{-n}$  under the full nonparametric model. The maximum empirical likelihood estimator (MELE) of  $F$  is  $\hat{F}(x) = \sum_{i=1}^n \hat{p}_i I(x \leq x_i) = n^{-1} \sum_{i=1}^n I(x \leq x_i)$ , where  $I(\cdot)$  denotes the indicator function.  $\hat{F}$  is none other than the ECDF  $F_n$  based on the i.i.d. sample  $\{x_1, x_2, \dots, x_n\}$ . Let  $\mu = EX_1$  and consider the hypothesis:

$$H_0 : \mu = \mu_0 \text{ vs. } H_1 : \mu \neq \mu_0.$$

The ELR is  $L(F)/L(F_n)$ . Following Owen (1988), the ELR at  $\mu$  can be written as

$$R(\mu) = \sup \left\{ \prod_{i=1}^n np_i : p_i \geq 0, \quad \sum_{i=1}^n p_i = 1, \quad \sum_{i=1}^n p_i x_i = \mu \right\}.$$

This optimization problem is solved by the Lagrange multipliers technique and Owen (1988) obtained the following result:

**Theorem 1.1.1.** (Owen, 1988) Assume  $EX_1^2 < \infty$ . Then under  $H_0$ ,

$$-2 \log R(\mu_0) \xrightarrow{\mathcal{D}} \chi_1^2, \quad \text{as } n \rightarrow \infty,$$

where  $\chi_1^2$  is a standard chi-squared random variable with one degree of freedom and  $\xrightarrow{\mathcal{D}}$  means converge in distribution.

Similar result has been obtained for random vectors according to the theorem:

**Theorem 1.1.2.** (Owen, 1988) Let  $\mathbf{X}_1, \mathbf{X}_2, \dots, \mathbf{X}_n$  be  $d \times 1$  i.i.d random vectors with distribution  $F$  with true mean  $\boldsymbol{\mu}_0$  and finite variance covariance matrix  $\boldsymbol{\Sigma}$  of rank  $q > 0$ . Then

$$-2 \log R(\boldsymbol{\mu}_0) \xrightarrow{\mathcal{D}} \chi_q^2, \quad \text{as } n \rightarrow \infty,$$

where  $\chi_q^2$  is a standard chi-squared random variable with  $q$  degrees of freedom.

A generalization to estimation equations is as follows. Let  $X_1, X_2, \dots, X_n$  be i.i.d. from a distribution  $F$ . Suppose a population parameter  $\boldsymbol{\theta}$  is determined by the equation  $E\{m(X_1, \boldsymbol{\theta})\} = 0$ , where  $\boldsymbol{\theta}$  is a  $q \times 1$  vector,  $m$  is a  $s \times 1$  vector-valued function. Suppose  $\boldsymbol{\theta}_0$  is the true value of  $\boldsymbol{\theta}$ . The ELR statistic to test  $H_0 : \boldsymbol{\theta} = \boldsymbol{\theta}_0$  is

$$R(\boldsymbol{\theta}_0) = \sup \left\{ \prod_{i=1}^n np_i : p_i \geq 0, \sum_{i=1}^n p_i = 1, \sum_{i=1}^n p_i m(X_i, \boldsymbol{\theta}_0) = 0 \right\},$$

and we have the following result:

**Theorem 1.1.3.** (Owen, 1990) Assume  $\text{Var}(m(X_1, \boldsymbol{\theta}_0)) < \infty$  and has rank  $r > 0$ . Then under  $H_0$ ,

$$-2 \log R(\boldsymbol{\theta}_0) \xrightarrow{\mathcal{D}} \chi_r^2, \quad \text{as } n \rightarrow \infty,$$

where  $\chi_r^2$  is a standard chi-squared random variable with  $r$  degrees of freedom.

**Remark 1.1.1.** For the proofs of all the above theorems, see the book "Empirical Likelihood" by Owen (2001).

Now, the EL has a vast domain of applications for complete and incomplete data and can handle a wide range of statistical parameters.

## 1.2 Survival Data

Survival data consists of data in which the time until the event (relapse, progression, death) is of interest. The response is often referred to as a failure time, survival time, or event time. Survival analysis provides methods and tools to analyze survival data. Fields like biomedical sciences (origin of survival data), engineering, economics, finance make use of survival analysis. Survival data are usually censored or incomplete. Censoring is present when we have some information about the subject's event time, but we do not know the

exact event time. Right censoring occurs when a subject does not experience the event before the study ends, is lost to follow-up during the study period, or withdraws from the study. Left-truncation occurs when a subject experienced the event before the study period. Figure 1.1 shows the different types of censoring.

Three topics in the present dissertation deal with survival data. For all the methods we discuss, the censoring mechanism is independent of the survival mechanism and is called independent censoring.

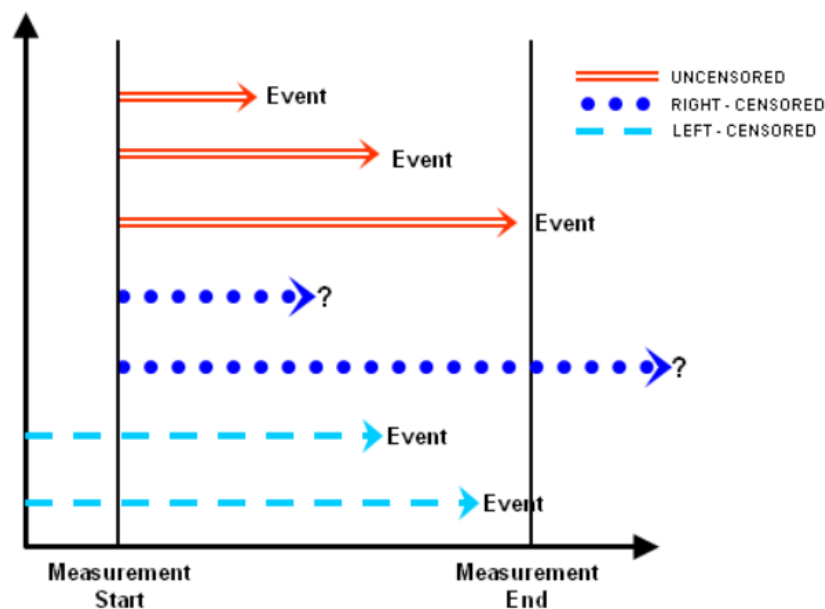


Figure 1.1: Different types of censoring.

### 1.3 Summary

As we pointed out in Section 1.1, EL has its advantages but also has its flaws. We explained how we solved some of these issues for specific parameters such as the Gini index, the mean residual life, the mean difference, and the Area Under the ROC Curve in the next lines.

The Gini index has been widely used as a measure of income (or wealth) inequality

in social sciences. To construct a confidence interval for the difference of two Gini indices from the paired samples, Wang and Zhao (2016) used a profile jackknife empirical likelihood. However, the computing cost with profile empirical likelihood could be very expensive. In the Chapter 2, we propose an alternative approach of the jackknife empirical likelihood method to reduce the computational cost. We also investigate the adjusted jackknife empirical likelihood and the bootstrap-calibrated jackknife empirical likelihood to improve coverage accuracy for small samples. Simulations show that the proposed methods perform better than Wang and Zhao's methods in terms of coverage accuracy and computational time. Real data applications demonstrate that the proposed methods work very well in practice. This chapter is the subject of an article published in the Journal of Nonparametric Statistics (<https://doi.org/10.1080/10485252.2019.1650925>).

The mean residual life (MRL) function for a given random variable  $T$  is the expected remaining lifetime of  $T$  after a fixed time point  $t$ . It is of great interest in survival analysis, reliability, actuarial applications, duration modeling, etc. Liang, Shen, and He (2016) proposed empirical likelihood (EL) confidence intervals for the MRL based on length-biased right-censored data. However, their  $-2\log(\text{empirical likelihood ratio})$  has a scaled chi-squared distribution. In Chapter 3, to avoid the estimation of the scale parameter in constructing confidence intervals, we propose a new empirical likelihood (NEL) based on independent and identically distributed (i.i.d.) representation of Kaplan-Meier weights involved in the estimating equation. We also established the adjusted new empirical likelihood (ANEL) to improve the coverage probability for small samples. The performance of the NEL and the ANEL compared to the existing EL is demonstrated via simulations: the NEL-based and ANEL-based confidence intervals have better coverage accuracy than the EL-based confidence intervals. Finally, our conclusions are illustrated with a real data set. This chapter produced an article in the Journal of Nonparametric Statistics (<https://doi.org/10.1080/10485252.2020.1840568>).

Chapter 4 focuses on comparing two means and finding a confidence interval for the difference of two means with right-censored data using the EL method combined with the independent and identically distributed (i.i.d.) random functions representation used by He

et al. (2016). In the literature, Wang and Wang (2001) proposed EL-based confidence intervals for the mean difference based on right-censored data using the synthetic data approach. However, their empirical log-likelihood ratio statistic has a scaled chi-squared distribution. To avoid the estimation of the scale parameter in constructing confidence intervals, we propose an EL method based on i.i.d. representation of Kaplan-Meier weights involved in the EL ratio. We obtain the standard chi-squared distribution. We also apply the adjusted empirical likelihood (AEL) to improve coverage accuracy for small samples. In addition, we investigate a new EL method, the mean empirical likelihood (MEL), within the framework of our study. The performances of all the EL methods are compared via extensive simulations. The proposed EL-based confidence interval has better coverage accuracy than these from Wang and Wang (2001). Finally, our findings are illustrated with a real data set.

The area under the ROC curve (AUC) gives an indicator of the quality of the prediction of a continuous-scale diagnostic test. It can also be used to compare the performance of two diagnostic tests. Chrzanowski (2014) proposed the weighted empirical likelihood (WEL) for interval estimation of a single AUC for right-censored data to reduce the computation associated with the usual empirical likelihood (EL) method. In Chapter 5, we propose the two-sample WEL to compare AUCs of two correlated ROC curves. A normal approximation (NA) is derived. We define a WEL ratio and show that the WEL statistic follows a scaled chi-square distribution. We also apply a calibration method, the adjusted empirical likelihood, to enhance the coverage accuracy of the Confidence interval (CI) for small samples. The good finite-sample performance of the proposed WEL is assessed via extensive simulations. Finally, the methodology is illustrated by a real example.

Chapter 6 summarizes the findings and proposals of the dissertation, while announcing the work and possible extensions to be considered in the future.



## CHAPTER 2

### REDUCE THE COMPUTATION IN JACKKNIFE EMPIRICAL LIKELIHOOD FOR COMPARING TWO CORRELATED GINI INDICES

#### 2.1 Background

Since its introduction in 1972 by the Italian statistician and sociologist, Corrado Gini, the Gini index has been one of the most used measures of the degree of inequality in the distribution of income and wealth. It has many applications in economics, statistics, medicine, and biology, etc. It is graphically related to the Lorenz curve, a very commonly used measure of the size of the distribution of income and wealth. The Lorenz curve is a plot of the cumulative proportion of total income (or wealth) received against the cumulative number of recipients, starting with the poorest recipient. Given a distribution, the Gini index is the ratio of the area between the Lorenz curve of the distribution and the uniform distribution line and the area under the uniform distribution line. It is also equal to half the relative mean difference. A Gini index of 0 indicates a perfect income equality and a Gini index of 1, a perfect inequality. Figure 5.1 shows the Gini index. For more details on the Gini index, the Lorenz curve and their properties, see Gastwirth (1972).

Let  $F(x) = P(X \leq x)$  be the cumulative distribution function of a non-negative random variable  $X$ . The Gini index  $G$  is defined as

$$G = \frac{1}{\mu} \int_0^{\infty} (2F(x) - 1)x dF(x) = \frac{E|X - Y|}{2EX}, \quad (2.1)$$

where  $X$  and  $Y$  are two independent random variables following the same distribution  $F(x)$  and  $\mu = EX$ .

For inference on the Gini index, parametric estimations have been adopted. The parameters of the distribution  $F$  of a random variable  $X$  are estimated for a chosen distribution,

and the Gini index is written as a function of these parameters, see for example Moothathu (1985, 1990) and Chotikapanich and Griffiths (2002). Bayesian estimations also have been considered by Abdul-Sathar, Jeevanand, and Nair (2005).

Nonparametric approaches focused on estimating the Gini index and the asymptotic variance of this estimate. Hoeffding (1948) first derived the asymptotic normality of the Gini index by estimating it as the ratio of two  $U$ -statistics. Ogowang (2000) introduced the variance estimation based on the ordinary least squares regression. This variance has been improved later by Giles (2004) and Modarres and Gastwirth (2006). Because implementing the estimates of the variance using the previous methods is complicated, Yitzhaki (1991) and Karagiannis and Kovacevic (2000) proposed the jackknife variance estimate for the Gini index. Davidson (2009) derived a reliable variance estimator of the plug-in estimator of the Gini index based on i.i.d. random variables.

Interval estimation of the Gini index can be obtained when the variance has been estimated. To avoid the estimation of the variance, some authors used bootstrap and empirical likelihood (EL) methods. Mills and Zandvakili (1997) and Biewen (2002) developed the naive bootstrap method. Davidson (2009) developed the bootstrap- $t$  method. Qin, Rao, and Wu (2010) introduced EL method, but their likelihood ratio test statistic was scale chi-square distributed and Peng (2011), by modifying the data, obtained the Wilks' theorem for the EL method. It has been shown that the EL confidence intervals outperformed normal approximation (NA) and bootstrap- $t$  confidence intervals. Wang, Zhao, and Gilmore (2016) derived the jackknife empirical likelihood (JEL) confidence interval for the Gini index and proved that the JEL performs better than the EL by Peng (2011). The JEL, proposed by Jing, Yuan, and Zhou (2009), basically turns the parameter of interest in a sample mean via pseudo-values and make the use of EL easier. Recently, Sang, Dang, and Zhao (2019) applied the JEL for inference on the Gini correlation, an useful measure in decomposition of the Gini index, according to income sources.

For comparing two Gini indices, Peng (2011) applied the EL method to obtain a confidence interval and compared it to Davidson's bootstrap- $t$  interval. Wang and Zhao (2016)

developed the JEL confidence intervals for the difference of two Gini indices for both independent and correlated data. It is worth mentioning that the JEL by Wang and Zhao (2016) has better coverage probability and shorter confidence interval than the EL by Peng (2011). The main goal of this chapter is to develop an alternative approach to the JEL method to reduce the computation associated with the JEL method proposed by Wang and Zhao (2016) for the paired samples.

The rest of the chapter is organized as follows. In Section 2.2, we introduce the notations and state the main asymptotic results. In Section 2.3, a simulation study is carried out to compare the proposed JEL methods with the JEL method from Wang and Zhao (2016) in terms of coverage probabilities and average lengths of confidence intervals. In Section 2.4, an application to two real data sets is provided, the conclusions are made in Section 2.5 and the proofs of theorems are given in the Appendix A.

## 2.2 Main Results

Given i.i.d. data set  $\mathbf{X} = \{X_1, X_2, \dots, X_n\}$ ,  $n \geq 2$ , the Gini index defined by equation (1) can be estimated by the ratio of two  $U$ -statistics with kernels  $h_1(x_1, x_2) = |x_1 - x_2|$  and  $h_2(x_1) = x_1$ , that is,

$$\hat{G} = \frac{U_1}{U_2} = \frac{\binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h_1(X_i, X_j)}{2n^{-1} \sum_{1 \leq i \leq n} h_2(X_i)}. \quad (2.2)$$

Based on  $U$ -statistics theory, Hoeffding (1948) derived the asymptotic normality of the estimator (2.2) for  $G$ . EL and JEL confidence intervals are proven to be efficient when compared to the normal approximation (NA) method. Therefore, we develop a modified version of the JEL method to compare two Gini indices from two dependent samples, in the following subsections.

### 2.2.1 Jackknife empirical likelihood (JEL)

To derive the proposed JEL confidence intervals for the difference of two correlated Gini indices for paired samples, we adopt the setting from Wang and Zhao (2016). Let

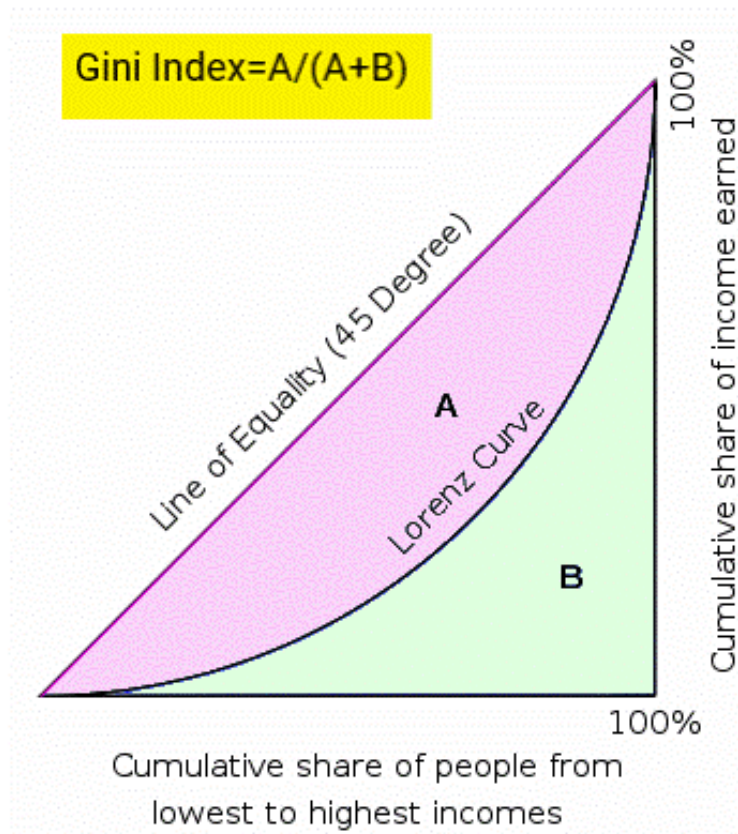


Figure 2.1: The Gini Index

$\{\mathbf{X}, \mathbf{Y}\}' = \{(X_1, Y_1)', \dots, (X_n, Y_n)'\}$  be i.i.d. bivariate random variables with common distribution function  $F(x, y)$ . Let  $F_1(x) = F(x, \infty)$  and  $F_2(y) = F(\infty, y)$  be the marginal distributions for  $X$  and  $Y$ , and  $G_1$  and  $G_2$  be the corresponding Gini indices associated with  $F_1(\cdot)$  and  $F_2(\cdot)$ , respectively.

Let  $\Delta = G_1 - G_2$ . Considering  $\Delta$  as the parameter of interest and  $G_2$  as a nuisance parameter, we define a vector of functional  $U$ -statistics as:

$$U'_n(\Delta, G_2) = \left( \binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h(X_i, X_j; \Delta + G_2), \binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h(Y_i, Y_j; G_2) \right),$$

with the kernel

$$h(x_1, x_2; G) = (x_1 + x_2)G - |x_1 - x_2|.$$

It is easy to check that  $Eh(X_1, X_2; \Delta + G_2 = 0)$  and  $Eh(Y_1, Y_2; G_2) = 0$ .

Since the Gini index is not a  $U$ -statistic, the results of Jing et al. (2009), applicable to one sample or two-sample  $U$ -statistics, cannot be applied directly for inference on the difference of two correlated Gini indices from paired samples, which is a function of two  $U$ -statistics and involves a nuisance parameter.

One approach to solve this issue is the profile JEL, developed by Wang and Zhao (2016), in which the estimated nuisance parameter minimizes the JEL ratio when  $\Delta$  is fixed. However, the computation of the profile JEL could be very costly, essentially due to difficulty in the maximization and moreover was made more complex in the case of two Gini indices as they involve a vector of  $U$ -statistics.

We can significantly reduce the computational burden by avoiding the optimization when the true value of  $G_2$  is known, but in practice, we ignore this value. We may choose to replace  $G_2$  by an estimate or plug-in estimator. The idea is not new. By using plug-in estimates of nuisance parameters in the estimating equations, Hjort, McKeague, and Van Keilegom (2009) reduced the computational burden, but their limiting distribution was a sum of weighted independent chi-squared random variables. Under the strong assumption that the kernel functions are bounded in both sample and parameter spaces, Li, Xu, and Zhou (2016) proposed inference procedure based on JEL for  $U$ -type estimating equations and were able to obtain the Wilks' theorem, but the Gini index requires only a finite second moment. Our approach uses a plug-in estimate and can establish the Wilks' theorem under this latter condition.

For the paired observations, since the equation

$$\binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h(Y_i, Y_j; G_2) = 0$$

does not depend on  $\Delta$ , it can be used to find an estimate  $\widehat{G}_2$  of  $G_2$ . Solving the above

equation, we obtain a closed form of  $\widehat{G}_2$ :

$$\widehat{G}_2 = \frac{\binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} |Y_i - Y_j|}{2\bar{Y}}, \quad (2.3)$$

where  $\bar{Y}$  is the sample mean. Then, plugging in  $\widehat{G}_2$  in  $h(x_i, x_j; \Delta + G_2)$ , we can apply the JEL method for  $\Delta$ . Let

$$M_n(\Delta) = \binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h(X_i, X_j; \Delta + \widehat{G}_2). \quad (2.4)$$

To apply JEL as defined by Jing et al. (2009), we define the jackknife pseudo-values as

$$\widehat{V}_i(\Delta) = nM_n(\Delta) - (n-1)M_n^{(-i)}(\Delta), \quad (2.5)$$

where

$$M_n^{(-i)}(\Delta) = \binom{n-1}{2}^{-1} \sum_{1 \leq k < j \leq n, k \neq i} h(X_k, X_j; \Delta + \widehat{G}_2)$$

is obtained after deleting the  $i^{\text{th}}$  observation  $X_i$  from the sample. As the jackknife pseudo-values are asymptotically independent under mild conditions (Shi 1984), the jackknife estimator  $M_{n,jack}$  can be viewed as a sample average of approximately independent random variables  $\widehat{V}_i$ . Then we can apply the standard EL method (Owen 2001) to  $\widehat{V}_i$ . Let  $P = \{p_1, p_2, \dots, p_n\}$  be the probability vector over the  $\widehat{V}_i$ . The JEL ratio at  $\Delta$  can be expressed as

$$R(\Delta) = \sup \left\{ \prod_{i=1}^n np_i : p_i \geq 0, \sum_{i=1}^n p_i = 1, \sum_{i=1}^n p_i \widehat{V}_i(\Delta) = 0 \right\}. \quad (2.6)$$

Applying the Lagrange multiplier technique,  $R(\Delta)$  is maximized at

$$p_i = \frac{1}{n\{1 + \lambda \widehat{V}_i(\Delta)\}}, \quad i = 1, \dots, n \quad (2.7)$$

where  $\lambda = \lambda(\Delta)$  satisfies

$$\frac{1}{n} \sum_{i=1}^n \frac{\widehat{V}_i(\Delta)}{1 + \lambda \widehat{V}_i(\Delta)} = 0. \quad (2.8)$$

Equation (2.8) can be solved with respect to  $\lambda$  by a simple grid search method or the NewtonRaphson procedure. By substituting  $\lambda$  in expression (2.7), we obtain  $p_i$ , knowing  $\widehat{V}_i(\Delta)$ , for  $i = 1, \dots, n$  and finally by replacing  $p_i$  in (2.6), we obtain the log-likelihood ratio

$$\log R(\Delta) = - \sum_{i=1}^n \log\{1 + \lambda \widehat{V}_i(\Delta)\}.$$

Let  $g(x; \Delta, G_2) = Eh(x, X_2; \Delta + G_2)$  and  $\sigma_g^2(\Delta, G_2) = Var(g(X_1; \Delta, G_2))$ . We have the Wilks' theorem for the JEL as follows.

**Theorem 2.2.1.** *Assume  $EX_1^2 < \infty$  and  $\sigma_g^2(\Delta, G_2) > 0$ . Then*

$$-2 \log R(\Delta) \xrightarrow{D} \chi_1^2, \quad \text{as } n \rightarrow \infty,$$

where  $\chi_1^2$  is a standard chi-squared random variable with one degree of freedom.

**Remark 2.2.1.** Due to the specific form of the Gini index, Theorem 2.2.1 requires finite second moment  $EX_1^2 < \infty$  instead of the regularity condition  $Eh^2(X_1, X_2) < \infty$  assumed in Theorem 1 of Jing et al. (2009) or the strong assumption on the kernels from Li et al. (2016).

**Remark 2.2.2.** Li et al. (2016) also proved that when nuisance parameters are replaced by plug-in estimates, the  $-2(\log\text{-likelihood ratio})$  is not a standard chi-square but a weighted chi-square distribution. However, we obtain the Wilks' theorem because in our particular case, the nuisance parameter  $G_2$  is estimated as a function of the parameter of interest  $\Delta$ , and the  $-2(\log\text{-likelihood ratio})$  involves only one chi-squared random variable with weight one (Li et al., 2011).

**Remark 2.2.3.** One advantage of the proposed method over JEL by Wang and Zhao (2016) and the EL by Peng (2011) is that it assumes only finite second moment whereas the last

two methods assume finite third moment. When applied to the income data, these methods could fail when the distributions are very skewed as the third moment may not exist.

A complete proof of Theorem 2.2.1 is given in the Appendix A. It involves additional modifications due to the estimator  $\widehat{G}_2$ .

Following this theorem, an asymptotic  $100(1 - \alpha)\%$  confidence interval for  $\Delta$  is given by

$$\{\tilde{\Delta} : -2 \log R(\tilde{\Delta}) \leq \chi_{1-\alpha}^2(1)\},$$

where  $\chi_{1-\alpha}^2(1)$  is the  $100(1 - \alpha)$  quantile of the chi-square distribution with one degree of freedom.

### 2.2.2 Adjusted jackknife empirical likelihood (AJEL)

The computation of  $\lambda$ , solution of (2.8) requires that the zero point is an interior point of the convex hull of the pseudo-values. However, when the sample size is small, this may not be the case and leads to under-coverage issue with the JEL. To resolve this problem for EL, Chen, Variyath, and Abraham (2008) proposed the adjusted empirical likelihood. We adapt this method to the JEL. To this end, we add one more pseudo-value

$$\widehat{V}_{n+1}(\Delta) = -\frac{a_n}{n} \sum_{i=1}^n \widehat{V}_i(\Delta),$$

where  $a_n = \max(1, \log(n)/2)$  as suggested by Chen et al. (2008). The adjusted jackknife empirical log-likelihood is defined as

$$\log R^A(\Delta) = -\sum_{i=1}^{n+1} \log\{1 + \lambda \widehat{V}_i(\Delta)\},$$

where  $\lambda = \lambda(\Delta)$  is the solution of

$$\frac{1}{n+1} \sum_{i=1}^{n+1} \frac{\widehat{V}_i(\Delta)}{1 + \lambda \widehat{V}_i(\Delta)} = 0. \quad (2.9)$$



We state Wilks' theorem for the AJEL as follows.

**Theorem 2.2.2.** *Assume  $EX_1^2 < \infty$  and  $\sigma_g^2(\Delta, G_2) > 0$ . Then*

$$-2 \log R^A(\Delta) \xrightarrow{D} \chi_1^2, \quad \text{as } n \rightarrow \infty.$$

Thus, an asymptotic  $100(1 - \alpha)\%$  AJEL confidence interval for  $\Delta$  is given by

$$\{\tilde{\Delta} : -2 \log R^A(\tilde{\Delta}) \leq \chi_{1-\alpha}^2(1)\}.$$

A sketch of the proof of Theorem 2.2.2 is given in the Appendix A.

### 2.2.3 Bootstrap calibration

The asymptotic distribution of the jackknife empirical log-likelihood ratio statistic is a chi-square distribution. When the sample size is small,  $\chi_{1-\alpha}^2(1)$  may be a poor critical value and consequently the under-coverage problem arises. To improve the coverage, we use the bootstrap-calibrated JEL according to the following procedure:

Step 1. Calculate  $\hat{G}_1$  and  $\hat{G}_2$  according to equations (2.2) and (2.3), respectively. Calculate  $\hat{\Delta} = \hat{G}_1 - \hat{G}_2$

Step 2. For  $b = 1, \dots, B$ ,

- Generate a bootstrap sample  $\{X_i^*, Y_i^*\}$ , of size  $n$ ,  $n \geq 2$ , from the original sample  $\{X_i, Y_i\}$  with replacement;  $i = 1, 2, \dots, n$ .
- Calculate the jackknife log-likelihood ratio  $\log R^B(\hat{\Delta})$  at  $\hat{\Delta}$  over the bootstrapped sample  $\{X_i^*, Y_i^*\}$ ;

Step 3. Calculate the bootstrap-calibrated  $100(1 - \alpha)\%$  JEL confidence interval for  $\Delta$  as

$$\{\tilde{\Delta} : -2 \log R(\tilde{\Delta}) \leq c_\alpha^*\},$$

where  $c_\alpha^*$  is the  $100(1 - \alpha)\%$  percentile of the  $B$  bootstrapped  $-2 \log R^B(\hat{\Delta})$ .

### 2.3 Simulation Study

Peng (2011) proved that EL-based methods have theoretical advantages over competing methods such as the percentile- $t$  bootstrap and NA, and Wang and Zhao (2016) demonstrated that the JEL outperformed the EL by Peng (2011). In this section, we report the results of a simulation study to compare the performance of the proposed JEL, adjusted JEL and the bootstrap-calibrated JEL methods named as JEL-AZ, AJEL-AZ and JEL-AZ-Boot, respectively with JEL, adjusted JEL and the bootstrap-calibrated JEL methods in Wang and Zhao (2016), denoted as JEL-WZ, AJEL-WZ and JEL-WZ-Boot, respectively.

The following scenarios have been used to generate paired data with a preselected correlation ( $\rho$ ):

S1:  $X \sim Uniform(0, 1)$  and  $Y \sim Uniform(0, 1)$ ,  $\rho = 0.80$ ,

S2:  $X \sim Exponential(2)$  and  $Y \sim Exponential(2)$ ,  $\rho = 0.80$ ,

S3:  $X \sim Weibull(0.5, 2)$  and  $Y \sim Weibull(0.5, 2)$ ,  $\rho = 0.75$ ,

S4:  $X \sim Lognormal(0, 1.5)$  and  $Y \sim Lognormal(0, 1.5)$ ,  $\rho = 0.85$ ,

S5:  $X \sim Gamma(7.7, 1)$  and  $Y \sim Uniform(1, 1.9)$ ,  $\rho = 0.75$ ,

S6:  $X \sim Lognormal(0, 1)$  and  $Y \sim Pareto(1.5, 3)$ ,  $\rho = 0.85$ .

The differences between the two correlated Gini indices are 0 for S1, S2, S3, S4, 0.1 for S5 and 0.321 for S6, respectively. The data in S1, S2, S3, and S4 have the skewnesses 0, 2, 4.12 and 33.84, respectively. We considered sample sizes 50, 100 and 150 with two nominal levels  $\alpha = 0.10$  and  $\alpha = 0.05$  for all the simulations. The process is repeated  $N = 2000$  times, and we used  $B = 1000$  replications for the bootstrap calibrations. Confidence intervals, average lengths of confidence intervals as well as the computation times to run the simulations are calculated for  $\Delta$  by JEL-WZ, AJEL-WZ, JEL-WZ-Boot, JEL-AZ, AJEL-AZ and JEL-AZ-Boot methods for all the simulated data. We also add the computation times of the EL by Peng, denoted EL-Peng. Literature shows that, for the paired samples, EL-Peng is a good competing nonparametric method for the JEL methods.

The simulation results are summarized in Tables 2.1-2.6 for coverage probability and

average lengths of confidence intervals. Tables 2.7-2.8 are for running times of the R codes used for simulations with each method. For reasons of brevity, these times are reported only for S1 and S2, but the results and conclusions are similar for S3-S6.

Table 2.1: Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods;  $X, Y \sim U(0, 1)$ .

	$n_1 = n_2 = 50$		$n_1 = n_2 = 100$		$n_1 = n_2 = 150$	
	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
JEL-WZ	0.884 (0.084)	0.940 (0.108)	0.889 (0.054)	0.942 (0.070)	0.897 (0.044)	0.948 (0.057)
JEL-AZ	0.903 (0.114)	0.942 (0.136)	0.895 (0.072)	0.949 (0.093)	0.898 (0.057)	0.948 (0.073)
AJEL-WZ	0.896 (0.089)	0.950 (0.116)	0.898 (0.061)	0.946 (0.080)	0.902 (0.049)	0.951 (0.061)
AJEL-AZ	0.928 (0.134)	0.966 (0.162)	0.929 (0.084)	0.966 (0.106)	0.922 (0.064)	0.960 (0.081)
JEL-WZ-Boot	0.914 (0.086)	0.957 (0.109)	0.893 (0.055)	0.951 (0.071)	0.895 (0.043)	0.949 (0.059)
JEL-AZ-Boot	0.905 (0.115)	0.953 (0.137)	0.899 (0.073)	0.943 (0.094)	0.898 (0.060)	0.950 (0.075)

Table 2.2: Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods;  $X, Y \sim exp(2)$ .

	$n_1 = n_2 = 50$		$n_1 = n_2 = 100$		$n_1 = n_2 = 150$	
	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
JEL-WZ	0.847 (0.087)	0.915 (0.112)	0.860 (0.058)	0.930 (0.076)	0.872 (0.044)	0.936 (0.060)
JEL-AZ	0.872 (0.139)	0.932 (0.167)	0.889 (0.093)	0.941 (0.116)	0.891 (0.070)	0.952 (0.091)
AJEL-WZ	0.863 (0.089)	0.936 (0.121)	0.868 (0.078)	0.937 (0.080)	0.878 (0.054)	0.942 (0.066)
AJEL-AZ	0.880 (0.176)	0.938 (0.215)	0.904 (0.115)	0.946 (0.140)	0.898 (0.081)	0.943 (0.107)
JEL-WZ-Boot	0.852 (0.091)	0.918 (0.112)	0.876 (0.069)	0.936 (0.094)	0.880 (0.049)	0.950 (0.071)
JEL-AZ-Boot	0.898 (0.148)	0.948 (0.180)	0.898 (0.096)	0.951 (0.122)	0.909 (0.073)	0.953 (0.099)

The following conclusions are made based on the tables.

- 1) All the coverage probabilities tend to their nominal levels (0.90 and 0.95) as the sample size increases. However JEL-AZ, AJEL-AZ and JEL-AZ-Boot converge faster than JEL-WZ, AJEL-WZ and JEL-WZ-Boot, respectively.
- 2) In general, JEL-AZ, AJEL-AZ and JEL-AZ-Boot based confidence intervals have better

Table 2.3: Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods;  $X, Y \sim Weibull(0.5, 2)$ .

	$n_1 = n_2 = 50$		$n_1 = n_2 = 100$		$n_1 = n_2 = 150$	
	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
JEL-WZ	0.823 (0.195)	0.898 (0.236)	0.858 (0.135)	0.910 (0.167)	0.850 (0.110)	0.916 (0.140)
JEL-AZ	0.840 (0.186)	0.901 (0.233)	0.850 (0.126)	0.902 (0.155)	0.852 (0.097)	0.913 (0.134)
AJEL-WZ	0.838 (0.196)	0.910 (0.239)	0.871 (0.135)	0.915 (0.168)	0.856 (0.114)	0.921 (0.148)
AJEL-AZ	0.865 (0.245)	0.907 (0.302)	0.868 (0.162)	0.935 (0.169)	0.876 (0.125)	0.935 (0.154)
JEL-WZ-Boot	0.863 (0.248)	0.916 (0.313)	0.867 (0.164)	0.925 (0.209)	0.881 (0.126)	0.938 (0.163)
JEL-AZ-Boot	0.865 (0.237)	0.922 (0.299)	0.865 (0.157)	0.923 (0.201)	0.878 (0.121)	0.929 (0.156)

Table 2.4: Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods;  $X, Y \sim Lognormal(0, 1.5)$ .

	$n_1 = n_2 = 50$		$n_1 = n_2 = 100$		$n_1 = n_2 = 150$	
	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
JEL-WZ	0.716 (0.211)	0.793 (0.256)	0.752 (0.164)	0.820 (0.202)	0.778 (0.139)	0.841 (0.173)
JEL-AZ	0.766 (0.193)	0.831 (0.247)	0.799 (0.165)	0.856 (0.207)	0.823 (0.149)	0.884 (0.183)
AJEL-WZ	0.736 (0.212)	0.815 (0.259)	0.760 (0.166)	0.833 (0.203)	0.783 (0.140)	0.845 (0.173)
AJEL-AZ	0.775 (0.280)	0.914 (0.348)	0.875 (0.202)	0.927 (0.258)	0.880 (0.170)	0.929 (0.205)
JEL-WZ-Boot	0.809 (0.334)	0.885 (0.440)	0.832 (0.254)	0.912 (0.328)	0.847 (0.197)	0.916 (0.255)
JEL-AZ-Boot	0.827 (0.434)	0.895 (0.593)	0.871 (0.297)	0.919 (0.398)	0.882 (0.242)	0.924 (0.312)

Table 2.5: Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods;  $X \sim Gamma(7.7, 1), Y \sim Uniform(1, 1.9)$ .

	$n_1 = n_2 = 50$		$n_1 = n_2 = 100$		$n_1 = n_2 = 150$	
	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
JEL-WZ	0.885 (0.048)	0.931 (0.062)	0.893 (0.033)	0.937 (0.043)	0.899 (0.027)	0.948 (0.035)
JEL-AZ	0.894 (0.058)	0.941 (0.074)	0.898 (0.039)	0.944 (0.051)	0.903 (0.032)	0.950 (0.042)
AJEL-WZ	0.900 (0.052)	0.943 (0.067)	0.901 (0.035)	0.944 (0.046)	0.906 (0.028)	0.951 (0.037)
AJEL-AZ	0.901 (0.070)	0.943 (0.090)	0.898 (0.045)	0.945 (0.058)	0.910 (0.036)	0.955 (0.046)
JEL-WZ-Boot	0.887 (0.049)	0.930 (0.064)	0.897 (0.034)	0.943 (0.045)	0.908 (0.028)	0.947 (0.036)
JEL-AZ-Boot	0.888 (0.058)	0.933 (0.076)	0.900 (0.041)	0.945 (0.053)	0.904 (0.033)	0.953 (0.042)

Table 2.6: Comparison of coverage probabilities and average lengths of the confidence intervals for different jackknife empirical likelihood methods;  $X \sim \text{Lognormal}(0, 1), Y \sim \text{Pareto}(1.5, 3)$ .

	$n_1 = n_2 = 50$		$n_1 = n_2 = 100$		$n_1 = n_2 = 150$	
	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
JEL-WZ	0.827 (0.173)	0.894 (0.210)	0.843 (0.122)	0.896 (0.151)	0.868 (0.101)	0.933 (0.128)
JEL-AZ	0.855 (0.181)	0.920 (0.227)	0.867 (0.131)	0.929 (0.162)	0.869 (0.106)	0.938 (0.135)
AJEL-WZ	0.836 (0.182)	0.903 (0.224)	0.848 (0.127)	0.908 (0.156)	0.878 (0.103)	0.933 (0.131)
AJEL-AZ	0.882 (0.221)	0.926 (0.244)	0.891 (0.150)	0.933 (0.184)	0.896 (0.116)	0.940 (0.150)
JEL-WZ-Boot	0.886 (0.237)	0.931 (0.298)	0.886 (0.155)	0.932 (0.195)	0.889 (0.125)	0.937 (0.158)
JEL-AZ-Boot	0.875 (0.271)	0.939 (0.343)	0.886 (0.172)	0.941 (0.221)	0.892 (0.133)	0.953 (0.183)

Table 2.7: Comparison of computing times (in seconds) for different empirical likelihood methods;  $X, Y \sim U(0, 1)$ .

	$n_1 = n_2 = 50$		$n_1 = n_2 = 100$		$n_1 = n_2 = 150$	
	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
EL-Peng	5453	6739	6918	6344	7007	7197
JEL-WZ	5294	6239	5998	6219	5513	6236
JEL-AZ	344	428	463	502	437	532
AJEL-WZ	3835	4991	4835	5603	4675	6114
AJEL-AZ	357	453	511	547	456	612
JEL-WZ-Boot	31460	31751	73171	67799	254544	220765
JEL-AZ-Boot	6131	7033	20735	19471	46992	44879

Table 2.8: Comparison of computing times (in seconds) for different empirical likelihood methods;  $X, Y \sim \text{exp}(2)$ .

	$n_1 = n_2 = 50$		$n_1 = n_2 = 100$		$n_1 = n_2 = 150$	
	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
EL-Peng	6757	7283	7977	9766	8947	13047
JEL-WZ	6143	6744	7597	8139	8687	12194
JEL-AZ	401	421	503	511	529	613
AJEL-WZ	10586	15713	11658	12080	12080	13421
AJEL-AZ	782	999	841	701	0817	1054
JEL-WZ-Boot	53337	51830	93824	97616	985437	1019975
JEL-AZ-Boot	9378	10070	45775	46053	95518	93067

coverage than these of the JEL-WZ, AJEL-WZ and JEL-WZ-Boot, respectively. The proposed intervals are wider than Wang and Zhao's when the distributions are non-skewed (S1) or moderately skewed (S2). They become comparable and even slightly shorter when the distributions are highly skewed (S3) or extremely skewed (S4).

- 3) For all the considered methods, the average lengths of the confidence intervals decrease when the sample sizes increase.
- 4) The computational times for EL-Peng and JEL-WZ methods are comparable with a slight advantage to the JEL-WZ method. The running times of JEL-WZ, AJEL-WZ and JEL-WZ-Boot simulations are 5 to 20 times these of JEL-AZ, AJEL-AZ and JEL-AZ-Boot's, respectively.
- 5) Both JEL methods faced low coverage probability problem for some scenarios (S3, S4 and S6) but as expected, AJEL and bootstrap calibration improved the coverage probability in all cases for small samples and fixed the under-coverage issue with bootstrap-calibration slightly better. However this latter is computationally time-consuming.

Based on the simulation results, we recommend the use of the proposed JEL-AZ method to obtain interval estimate for the difference of two correlated Gini indices and its AJEL-AZ for small samples or the bootstrap-calibrated JEL-AZ-Boot when the time is not an issue.

## 2.4 Real Applications

In this section, we apply the proposed methods to two real data sets. First, the data are extracted from the Penn World Tables (PWT) database. Since 1950, PWT gathered information on the production, income, and prices to measure real gross domestic product (GDP) across countries over time, for 182 countries. A complete description of this data set can be found in Summers and Heston (1995). We used it to compare the estimates of the real GDP per capita in constant dollars expressed in international prices, base year 1985, for the years 1970 and 1990. In this case, the Gini index measures the dispersion of real

GDP across the 108 countries for which data are available. Figure 2.2 shows histograms and Lorenz curves for the data at years 1970 and 1990 and the scatter plot between them. We notice that both data from years 1970 and 1990 seem to be exponentially distributed and their estimated Gini indexes (0.488 and 0.525) are very close to 0.5, the Gini index of the exponential distribution. The data from both years are highly correlated ( $\rho = 0.93$ ) and moderately skewed with the skewnesses 1.33 and 1.12, respectively for the years 1970 and 1990. We calculate the confidence interval for the difference between the Gini indices at years 1970 and 1990, at the 90% and 95% levels using our JEL methods and JEL methods from Wang and Zhao (2016). The difference is obtained using the unbiased estimates (via  $U$ -statistics), and the biased estimates (via plugging in the empirical distribution function) of the Gini indices and the results are summarized in Table 2.9.

The study confirms that JEL-AZ confidence intervals lengths for the difference between the Gini indices at years 1970 and 1990 are wider than these of JEL-WZ, which is consistent with the simulations results according to the skewness of the data (see Table 2.1 and Table 2.2 for non-skewed or moderately skewed data). But in both cases, the confidence intervals are small enough to conclude that there is a significant difference between the two indices ( $G_1 < G_2$ ).

Table 2.9: Point and interval estimates for the parameters of interest from the PWT data set.

	Point estimate			Interval estimate	
	$G_1$ at 1970	$G_2$ at 1990	$G_1 - G_2$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
Plug-in	0.484	0.520	-0.037	JEL-WZ (-0.060, -0.011)	(-0.056, -0.015)
$U$ -statistic	0.488	0.525	-0.037	JEL-AZ (-0.071, -0.001)	(-0.066, -0.007)
				AJEL-WZ (-0.059, -0.010)	(-0.055, -0.015)
				AJEL-AZ (-0.110, -0.010)	(-0.098, -0.017)

Second, we consider the Panel Study of Income Dynamics (PSID) data, a longitudinal panel survey of American families with a sample of over 18,000 individuals in 5,000 households. The study is directed by the Survey Research Center at the University of Michigan

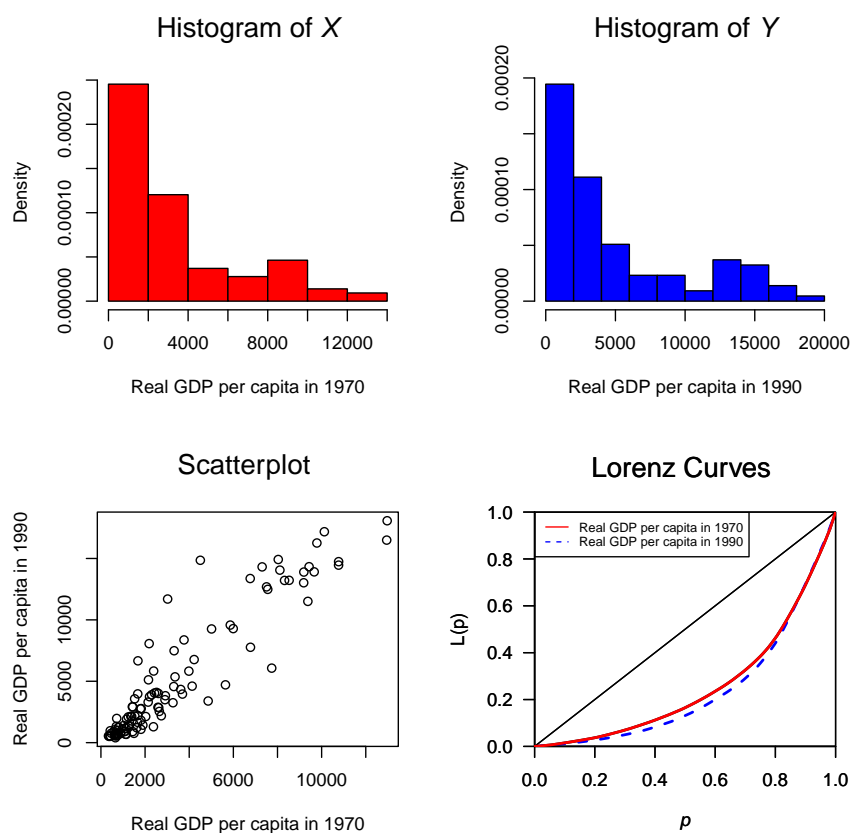


Figure 2.2: Exploratory plots for the PWT data set.

since 1968, and the data encompass many health, social, and economic factors. The data can be downloaded directly from the PSID website (<https://psidonline.isr.umich.edu/>). Here, the Gini index is calculated directly based on the total family income, and we are interested in the difference in the Gini indices for the years 1996 and 1998. These indices are 0.450 and 0.457, respectively. Figure 2.3 shows histograms and Lorenz curves as well as the scatter plot of the data for years 1996 and 1998. The observations are strongly correlated ( $\rho = 0.70$ ) and very skewed. The skewnesses are 4.58 and 8.98 for the years 1996 and 1998, respectively. One can hardly detect the difference between the two Lorenz Curves. As before, we compute the confidence interval for the difference between the Gini indices at years 1996 and 1998, at the 90% and 95% levels by different JEL methods. Table 2.10 shows the results of



the application. While all the methods conclude there is no significant difference between the two Gini indices ( $G_1 = G_2$ ), the proposed methods result in slightly shorter confidence intervals in concordance with the simulation conclusions in Table 2.3 and Table 2.4 for the very skewed data.

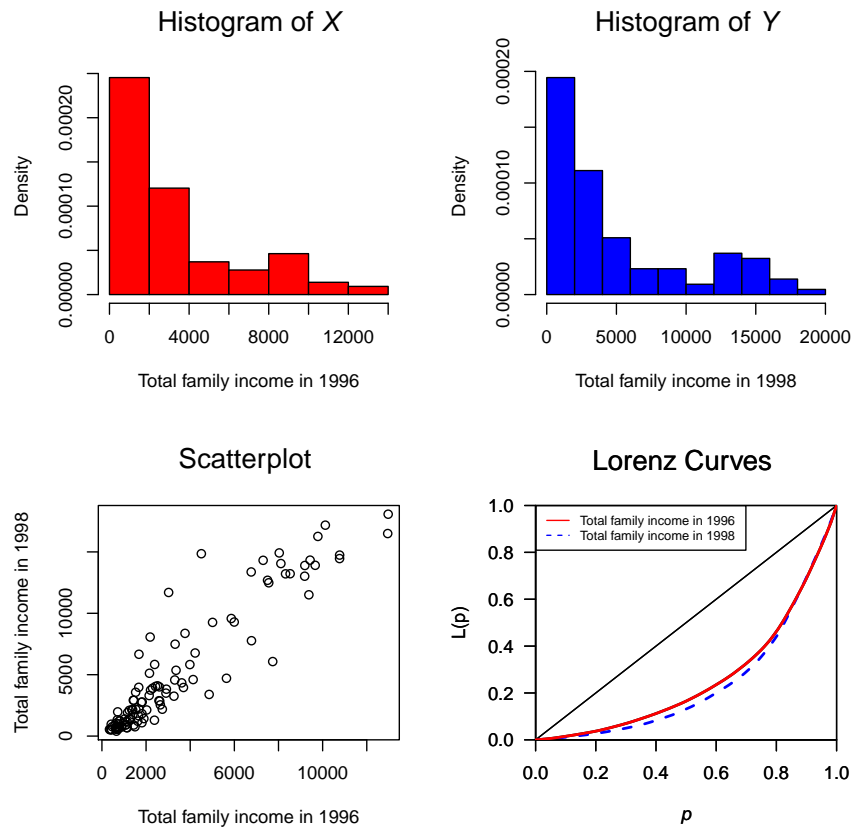


Figure 2.3: Exploratory plots for the PSID data set.

## 2.5 Conclusions

In this chapter, we have considered new jackknife empirical likelihood confidence intervals for the difference between two correlated Gini indices based on the paired observations. We have shown that, under some weak conditions, the log-likelihood ratio statistic converges to a chi-squared distribution. A confidence interval is then constructed for the difference

Table 2.10: Point and interval estimates for the parameters of interest from the PSID data set.

	Point estimate			Interval estimate	
	$G_1$ at 1996	$G_2$ at 1998	$G_1 - G_2$	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
Plug-in	0.451	0.458	-0.007	JEL-WZ (-0.017, 0.002)	(-0.019, 0.003)
$U$ -statistic	0.450	0.457	-0.007	JEL-AZ (-0.015, 0.002)	(-0.016, 0.004)
				AJEL-WZ (-0.017, 0.002)	(-0.019, 0.003)
				AJEL-AZ (-0.015, 0.002)	(-0.017, 0.004)

between two correlated Gini indices by using the proposed method, and compared with the jackknife empirical likelihood-based methods from Wang and Zhao (2016) via extensive simulation studies. Not only do the confidence intervals tend to the nominal level when the sample size increases for both methods but also our JEL-AZ methods outperform the JEL-WZ methods in terms of coverage probability in all cases. Moreover, our JEL-AZ methods are 5 to 20 times faster than JEL-WZ methods, which allows us to save a lot of computational time. Finally, real applications are given to illustrate the performance of the proposed methods.

## CHAPTER 3

### NEW EMPIRICAL LIKELIHOOD INFERENCE FOR THE MEAN RESIDUAL LIFE WITH LENGTH-BIASED AND RIGHT-CENSORED DATA

#### 3.1 Background

The mean residual life function (MRL) of a non-negative continuous random variable  $T$  at time  $t$  is defined as

$$m(t) = E(T - t | T > t) = \frac{\int_t^\infty S(u) du}{S(t)} I(S(t) > 0),$$

where  $S(t)$  denotes the survival function of  $T$  and  $I(\cdot)$  is the indicator function. The MRL exists if and only if  $m(0) = E(T)$ . The mean residual life function is an important characteristic of the survival time as it plays the same role as a probability density function (PDF), a cumulative distribution function (CDF), or a survival function. Because the MRL function completely characterizes a lifetime, statisticians have studied its statistical inference for a long time. It is in this way that Yang (1977) constructed an estimator for  $m(t)$  with censored data based on Kaplan-Meier estimates. Yang (1978) considered an empirical estimate of  $m(t)$  on a finite sample and proved that it is strongly consistent and converges in distribution to a Gaussian process. Kumazawa (1987) showed the consistency of the estimator of  $m(t)$  proposed by Yang (1977) with right-censored data and proved that it converges weakly to a Gaussian process on the whole line. Oakes and Dasu (1990) proposed the proportional mean residual life model. Csörgő and Zitikis (1996) proposed a nonparametric estimator and constructed a normal approximation (NA)-based confidence intervals of the MRL based on i.i.d. complete observed lifetimes. Chen and Cheng (2006) and Chen (2007) developed

the additive mean residual life model. Sun and Zhang (2009) extended the proportional and additive MRL and introduced a class of transformed  $m(t)$  to fit survival data under right censoring. Zhao and Qin (2006) and Qin and Zhao (2007), developed empirical likelihood (EL) inference of the MRL for complete i.i.d. data and right censored data and showed through simulation studies that the EL method performs better than the normal approximation method. More recently, Zhao, Jiang, and Liu (2013) proposed an estimation method of the MRL with left truncated and right-censored data and showed that the proposed estimator converges weakly to a Gaussian process.

Length bias is a form of selection bias. It occurs when the probability of sampling a random variable is proportional to its length (Cox, 1969) or when the random variable is subject to left truncation with the truncation variables independent and uniformly distributed on a well-defined interval: it is called the stationary assumption (Wang, 1991). It often arises when prevalent sampling is used for recruiting cohort subjects as the subjects that have already experienced the event of interest and have been recruited after the onset time are often excluded from the study. Statistical inferences of length-biased data also have been studied by statisticians. For example, Vardi (1982) derived a nonparametric maximum likelihood estimator of a lifetime distribution  $F$  based on two samples, one from  $F$ , the other from the length-biased distribution of  $F$  and proved that it converges to a pinned Gaussian process. Gupta and Keating (1986) demonstrated the unique relationships between the corresponding reliability measures (i.e., the survival functions, hazard functions, and mean residual life functions) of a distribution and its length-biased version. Huang and Qin (2012) proposed a composite partial likelihood method for the Cox model with survival data collected under length-biased sampling to study the survival between the vascular dementia group and the possible Alzheimer's disease group for the Canadian Study of Health and Aging (CSHA) data. Recently, Li, Ma, and Wang (2017) proposed a semi-parametric method to analyze general biased data under the additive risk model by estimating the regression parameter and the non-parametric function. They proved the consistency and asymptotic normality of the estimators for length-biased data, and they did not need the information about the

truncation time.

Many other studies involve the MRL function. For example, Chan, Chen, and Di (2012), to study disease associations with risk factors in epidemiological studies, applied the proportional mean residual life model of Oakes and Dasu (1990) to censored length-biased survival data. Ning, Qin, Asgharian, and Shen (2013) proposed a constrained EM algorithm to derive nonparametric confidence intervals based on an EL ratio for length-biased right-censored data. Wu and Luan (2014) proposed an efficient estimator of the MRL with length-biased and right-censored data and proved that it converges to a zero-mean normal distribution. Fakoor (2015) developed a nonparametric estimator of the MRL based on estimating the distribution function of the length-biased lifetime, and the estimate converges to a mean-zero Gaussian process. In the presence of right censoring, the limiting distribution of the EL based log-likelihood ratio is a scaled chi-square distribution (Qin and Zhao, 2007). He, Liang, Shen, and Yang (2016) proposed influence functions in an estimating equation and showed that under very general conditions,  $-2\log(\text{EL ratio})$  converges weakly to a standard chi-square distribution. Similar idea also can be found in Sun et al. (2009) for confidence regions for the time-dependent regression coefficients in Cox regression models; in Zhao and Huang (2007) and Wu et al. (2015) for interval estimate of the parameter in AFT models; in Zhao and Jinnah (2012) to estimate the unspecified baseline hazard function and regression parameters in the Cox regression model; in Zhao and Yang (2012) for EL inference on the regression parameters of the survival rate while avoiding a covariance matrix and in Huang and Zhao (2018) for inference on the bivariate survival function, etc. Recently, Liang et al. (2016), based on the LR method from Murphy and van der Vaart (1997), proposed an EM-algorithm to calculate the likelihood ratio directly for length-biased and right-censored data and proved that the corresponding log-likelihood ratio converges to the standard chi-square distribution.

To construct confidence intervals for the MRL based on length-biased right-censored data, the  $-2\log(\text{EL ratio})$  proposed by Liang et al. (2016) has a scaled chi-squared distribution. The scale parameter, which is a function of the asymptotic variance must be estimated.

This fact removes one of the benefits that the EL has over the NA; that is, EL does not need a variance estimation.

In this chapter, we propose a new empirical likelihood (NEL) inference procedure for the MRL with length-biased and right-censored data and show that, under some regularity conditions, the limiting distribution of the empirical log-likelihood ratio for the MRL is a standard chi-square distribution and by doing so we avoid the estimation of the scale parameter from Liang et al. (2016). The asymptotic property is then used to construct NEL-based confidence interval for the MRL. Moreover, we develop the adjusted new empirical likelihood (ANEL) for the MRL function to solve the convex hull problem encountered in the EL. Simulations showed that our proposed NEL-based and ANEL-based intervals have better coverage accuracy than the scaled EL intervals but slightly longer widths.

The rest of the chapter is organized as follows. In Section 3.2, we introduce the notations and state the main asymptotic result. In Section 3.3, a simulation study is carried out to compare the proposed NEL and ANEL methods with the EL method from Liang et al. (2016) in terms of coverage probability and average length of confidence interval. In Section 3.4, an application to the Channing House data set is provided, and the conclusions are made in Section 3.5. The proofs of Theorems are given in the Appendix B.

## 3.2 Main Results

### 3.2.1 New empirical likelihood (NEL)

Following the set up in Liang et al. (2016), let  $\{T_1, T_2, \dots, T_n\}$  be i.i.d. positive random variables with a common CDF  $F$  ( $T$  represents the true failure time variable). When a non-negative random variable  $Y$  is observed with probability proportional to its length (Cox, 1969), it has the length-bias CDF

$$L_F(y) = \frac{1}{\mu} \int_0^y x dF(x), \quad y \geq 0$$

where  $\mu = ET = \int_0^\infty S(t)d(t)$  is finite and  $S = 1 - F$  is the survival function of  $T$ .

$L_F$  can be seen as the CDF of a randomly left truncated random variable in the stationary case (Wang 1991). Let  $A$  be the left truncated observed variable.  $A$  is uniformly distributed on  $[0, Y]$  and has the survival function

$$P(A > x) = \frac{1}{\mu} \int_x^\infty S(u)d(u).$$

Let  $\tau_F = \inf\{t : F(t) = 1\}$  be the upper bound of  $T$ . The residual survival life is  $R = Y - A$  and the MRL  $m(t_0) = E(R|A > t_0)$  at time  $t_0$  becomes

$$m(t_0) = \frac{E(RI(A > t_0))}{P(A > t_0)} = \frac{\int_{t_0}^\infty uS(u)d(u)}{\int_{t_0}^\infty S(u)d(u)} - t_0,$$

$$m(t_0) = \frac{\int_{t_0}^{\tau_F} uS(u)d(u)}{\int_{t_0}^{\tau_F} S(u)d(u)} - t_0 = \frac{E(T - t_0)^2 I(T > t_0)}{2E(T - t_0) I(T > t_0)}.$$

For the rest of the paper, we denote  $m(t_0)$  by  $m$ . The last equality leads to the estimation equation:

$$E [2(T - t_0) m - (T - t_0)^2] I(T > t_0) = 0.$$

Let  $\{C_1, C_2, \dots, C_n\}$  be i.i.d. random variables with common CDF  $G$ , representing the censoring variable. Suppose that  $T$  and  $C$  are independent. We observe that:

$$Z_i = \min(T_i, C_i), \quad \delta_i = I(T_i \leq C_i), \quad i = 1, 2, \dots, n.$$

The distribution  $H$  of  $Z$  satisfies  $(1 - H) = (1 - F)(1 - G)$ . Let  $\{Z_{(1)}, Z_{(2)}, \dots, Z_{(n)}\}$  be the ordered values of  $Z$  and  $\{\delta_{(1)}, \delta_{(2)}, \dots, \delta_{(n)}\}$  the corresponding values of  $\delta$  associated to  $Z_{(i)}$ , i.e., the  $i$ -th concomitant. The estimating equation becomes:

$$E \frac{\delta}{1 - G(Z)} (2(Z - t_0) m - (Z - t_0)^2) I(Z > t_0) = 0,$$

as established in Liang et al. (2016). Since  $G$  is unknown, it is replaced by its Kaplan-Meier estimator  $\widehat{G}_n$ ,

$$\widehat{G}_n(t) = 1 - \prod_{i=1}^n \left[ \frac{n-i}{n-i+1} \right]^{I(Z_{(i)} \leq t, \delta_{(i)}=0)},$$

and the proposed estimating equation finally becomes

$$U(m) = \sum_{i=1}^n V_{ni}(m) = \sum_{i=1}^n \frac{2(Z_i - t_0)m - (Z_i - t_0)^2}{1 - \widehat{G}_n(Z_i)} I(Z_i > t_0) \delta_i = 0, \quad (3.1)$$

from which EL confidence intervals are derived for the MRL. However, this EL ratio converges to a scaled  $\chi_1^2$  distribution. The scale parameter is a function of an unknown asymptotic variance, which has been estimated using the complicated jackknife estimator of the asymptotic variance proposed by Stute (1996). Even though any other variance estimator could be used, the method is time-consuming. One can notice that Equation (1) involves the Kaplan-Meier weights  $(1 - \widehat{G}_n(Z_i))^{-1}$ . Many authors have obtained an i.i.d. representation of Kaplan-Meier estimator, which will be used to obtain our main result.

Suppose that  $m_0$  is the true value of  $m$ . By replacing  $(1 - \widehat{G}_n(Z_i))^{-1}$  in  $V_{ni}(m)$  (see Equation (1)) by the i.i.d. representation from He and Huang (2003) (see Lemma 3.1) and using the counting process notation, we let:

$$W_i(m) = \frac{\Phi(Z_i, m) \delta_i}{1 - G(Z_i)} + \int_0^\infty \frac{\psi(s, m)}{\overline{H}(s)} (dN_i^C(s) - Y_i(s) d\Lambda^C(s)), \quad (3.2)$$

where  $\Phi(t, m) = \{2(t - t_0)m - (t - t_0)^2\}I(t > t_0)$ ,  $\psi(s, m) = \int_s^\infty \Phi(x, m) dF(x)$ ,  $H(s) = EI(Z_i \leq s)$ ,  $N_i^C(s) = I(Z_i \leq s, \delta_i = 0)$ ,  $Y_i(s) = I(Z_i \geq s)$  and  $\Lambda^C(s) = -\log(1 - G(s))$  is the cumulative hazard function of  $C$ .

$U(m_0)$  is asymptotically equivalent to  $\sum_{i=1}^n W_i(m_0)$ , where  $W_i(m_0)$  are i.i.d. random variables with mean zero for  $i = 1, \dots, n$  in the sense that

$$U(m_0) = \sum_{i=1}^n W_i(m_0) + o_p(n^{1/2}).$$



Motivated by this i.i.d. representation, we define

$$W_{ni}(m) = \frac{\Phi(Z_i, m) \delta_i}{1 - \widehat{G}_n(Z_i)} + \int_0^\infty \frac{\sum_{j=1}^n \omega_{(j)} I(Z_{(j)} \geq s) \Phi(Z_{(j)}, m)}{n^{-1} \sum_{j=1}^n I(Z_j \geq s)} \times \left[ dN_i^C(s) - Y_i(s) d\widehat{\Lambda}^C(s) \right], \quad (3.3)$$

by replacing  $G$  by  $\widehat{G}_n$ ,  $H(s)$  by  $n^{-1} \sum_{j=1}^n I(Z_j \leq s)$ ,  $\psi(s, m)$  by  $\sum_{j=1}^n \omega_{(j)} I(Z_{(j)} \geq s) \Phi(Z_{(j)}, m)$

and  $d\Lambda^C(s)$  by  $d\widehat{\Lambda}^C(s) = dN^C(s)/Y(s)$  in  $W_i(m)$ , where  $N^C(s) = \sum_{i=1}^n N_i^C(s)$ ,  $Y(s) =$

$\sum_{i=1}^n Y_i(s)$  and  $\omega_{(j)} = \frac{\delta_{(j)}}{n-j+1} \prod_{k=1}^{j-1} \left[ \frac{n-k}{n-k+1} \right]^{\delta_{(k)}}$  for  $j = 1, 2, \dots, n$  (see Stute, 1995). Based on  $W_{ni}(m)$ 's, we define the estimated  $EL$  ratio at the value  $m$  (cf. Owen 1988, 1990) as follows

$$R(m) = \sup \left\{ \prod_{i=1}^n n p_i : \sum_{i=1}^n p_i = 1, \sum_{i=1}^n p_i W_{ni}(m) = 0, p_i \geq 0 \right\}.$$

By the technique of Lagrange multipliers, it is easy to show that

$$l(m) = -2 \log R(m) = 2 \sum_{i=1}^n \log \{1 + \lambda W_{ni}(m)\},$$

where  $\lambda = \lambda(m)$  satisfies the equation

$$\frac{1}{n} \sum_{i=1}^n \frac{W_{ni}(m)}{1 + \lambda W_{ni}(m)} = 0. \quad (3.4)$$

Although  $W_{ni}(m)$ 's are not independent, they can be used to construct empirical likelihood ratio and obtain the usual standard  $\chi_1^2$  distribution asymptotically, according to the following Wilks theorem.

**Theorem 3.2.1.** *Let  $m_0$  be the true value of  $m$ . Assume that the regularity conditions in*

the Appendix hold. Then

$$l(m_0) \xrightarrow{D} \chi_1^2, \quad \text{as } n \rightarrow \infty,$$

where  $\chi_1^2$  is a standard chi-squared random variable with one degree of freedom.

Thus, using Theorem 3.2.1, an asymptotic  $100(1 - \alpha)\%$  EL confidence interval for  $m$  is given by

$$I = \{m : l(m) \leq \chi_1^2(\alpha)\},$$

where  $\chi_1^2(\alpha)$  is the upper  $\alpha$ -quantile of the distribution of  $\chi_1^2$ .

### 3.2.2 Adjusted new empirical likelihood (ANEL)

Chen, Variyath, and Abraham (2008) introduced the adjusted empirical likelihood (AEL) to solve the under-coverage problem encountered by the EL method for small samples. The key idea of the AEL is to add an observation to the data to ensure that the convex hull of the  $W_{ni}(m)$  always contains zero. By doing so, they solve the empty set problem and the AEL is well defined for all  $m$ . This technique is coupled with the NEL to obtain the adjusted new empirical likelihood (ANEL). To apply the ANEL, we add a pseudo-data  $W_{nn+1}(m)$  to the sample

$$W_{nn+1}(m) = -\frac{a_n}{n} \sum_{i=1}^n W_{ni}(m),$$

where  $a_n = \max(1, \log(n)/2)$  as suggested by Chen et al. (2008). Based on the  $n + 1$  observations, we define the adjusted new empirical log-likelihood ratio as

$$R^A(m) = \sup \left\{ \prod_{i=1}^{n+1} np_i : \sum_{i=1}^{n+1} p_i = 1, \sum_{i=1}^{n+1} p_i W_{ni}(m) = 0, p_i \geq 0 \right\}.$$

Using the method of Lagrange multipliers, we can show that

$$l^A(m) = -2 \log R^A(m) = 2 \sum_{i=1}^{n+1} \log\{1 + \lambda^A W_{ni}(m)\},$$

where  $\lambda^A = \lambda^A(m)$ , the Lagrange multiplier, is a solution of the equation

$$\frac{1}{n+1} \sum_{i=1}^{n+1} \frac{W_{ni}(m)}{1 + \lambda^A W_{ni}(m)} = 0. \quad (3.5)$$

The ANEL retains Wilks' theorem as follows.

**Theorem 3.2.2.** *Assume that the regularity conditions in the Appendix hold. Then*

$$l^A(m_0) \xrightarrow{\mathcal{D}} \chi_1^2, \quad \text{as } n \rightarrow \infty.$$

Thus, using Theorem 3.2.2, an asymptotic  $100(1 - \alpha)\%$  ANEL confidence interval for  $m$  is constructed as follows

$$I^A = \{m : l^A(m) \leq \chi_1^2(\alpha)\}.$$

### 3.3 Simulation Study

In this section, we report the results of a simulation study to compare the finite-sample performance of the new empirical likelihood method (NEL) and its adjustment (ANEL) with the existing empirical likelihood (EL) method in Liang et al. (2016). Once the lifetime  $T$  is generated, the left-truncated variable  $A$  is uniformly distributed with an upper bound larger than the upper bound of  $T$ , to ensure the stationary assumption. We generate pairs of observations  $(A, T)$  until we obtain  $n$  pairs satisfying  $(A \leq T)$ . Then, all is right-censored by a variable  $C$ . We consider the following cases for the simulated data:

S1:  $T \sim \text{Uniform}(0, 1)$ ,  $A \sim \text{Uniform}(0, 10)$ , and  $C \sim \text{Uniform}(0, c)$ ,

S2:  $T \sim \text{Weibull}(2, 1/\sqrt{2})$ ,  $A \sim \text{Uniform}(0, 15)$ , and  $C \sim \text{Exponential}(\lambda)$ ,

S3:  $T \sim \text{Lognormal}(2, 1/2)$ ,  $A \sim \text{Uniform}(0, 35)$ , and  $C \sim \text{Exponential}(\lambda)$ ,

where  $c$  and  $\lambda$  are chosen to control the censoring proportion. The true values of the length-

biased mean residual life function at given time  $t_0$  are

$$m(t_0) = \frac{1 - t_0}{3} I(0 \leq t_0 \leq 1),$$

$$m(t_0) = \frac{e^{-\frac{t_0^2}{2}}}{\sqrt{\frac{\pi}{2}} \operatorname{erfc}\left(\frac{t_0}{\sqrt{2}}\right)} - t_0,$$

$$m(t_0) = \frac{\frac{e^5}{2} \operatorname{erfc}(\ln(t_0) - 3) - \frac{t_0^2}{2} \operatorname{erfc}(\ln(t_0) - 2)}{e^{\frac{9}{4}} \operatorname{erfc}\left(\ln(t_0) - \frac{5}{2}\right) - t_0 \operatorname{erfc}(\ln(t_0) - 2)} - t_0,$$

for S1, S2 and S3 respectively, where *erfc* is the complementary error function.

Based on the simulated data set, the EL-based, NEL-based and ANEL-based confidence intervals and average lengths are calculated according to Theorem 3.1 (Liang et al., 2016), Theorem 3.2.1, and Theorem 3.2.2 in Section 3.2 for 90% and 95% confidence levels. For each fixed value of  $c$ ,  $\lambda$ , and sample size  $n$ , the process is repeated 5000 times. The coverage probabilities and average lengths of confidence intervals are calculated at  $t_0 = 0.1, 0.3, 0.5, 0.7$  for S1, at  $t_0 = 0.5, 0.75, 1, 1.5$  for S2, and at  $t_0 = 1, 2, 3, 4$  for S3, respectively.

For different values of  $c$  and  $\lambda$ , 10%, and 30% censoring proportions are achieved. The simulation results are summarized in Tables 3.1, 3.2, 3.7 for S1, Tables 3.3 and 3.4 for S2, and Tables 3.5 and 3.6 for S3.

Based on the tables we can make the following conclusions:

- 1) All the coverage probabilities tend to their nominal levels (0.90 and 0.95) as the sample size increases. They are close to the nominal levels when  $t_0$  is small. They start to decrease as  $t_0$  increases, essentially because there is less information in the data, the MRL at  $t_0$  being defined for values of the sample greater than  $t_0$ . The best coverage occurs often when  $t_0$  is small.
- 2) For all censoring rates (10%, 30%), NEL-based confidence intervals perform better than those of the EL-based confidence intervals.
- 3) For  $n \geq 150$ , NEL-based confidence intervals attain the fixed nominal levels, but are

Table 3.1: Comparison of coverage probabilities (average lengths) of the confidence intervals with 10% censoring rate, for S1.

Nominal level	Sample size ( $n$ )	Method	$t_0=0.1$	$t_0=0.3$	$t_0=0.5$	$t_0=0.7$
0.90	50	EL	0.888 (0.0483)	0.887 (0.0428)	0.878 (0.0356)	0.845 (0.0253)
		NEL	0.898 (0.0505)	0.894 (0.0442)	0.885 (0.0365)	0.846 (0.0256)
		ANEL	0.910 (0.0556)	0.903 (0.0512)	0.890 (0.0415)	0.861 (0.0310)
	100	EL	0.891 (0.0339)	0.889 (0.0298)	0.880 (0.0249)	0.863 (0.0193)
		NEL	0.899 (0.0354)	0.892 (0.0308)	0.885 (0.0256)	0.871 (0.0196)
		ANEL	0.907 (0.0434)	0.898 (0.0408)	0.900 (0.0319)	0.884 (0.0218)
	150	EL	0.899 (0.0279)	0.897 (0.0248)	0.888 (0.0206)	0.879 (0.0158)
		NEL	0.901 (0.0290)	0.900 (0.0255)	0.891 (0.0210)	0.889 (0.0160)
		ANEL	0.916 (0.0335)	0.905 (0.0305)	0.902 (0.0254)	0.896 (0.0217)
	200	EL	0.901 (0.0240)	0.899 (0.0211)	0.893 (0.0183)	0.891 (0.0141)
		NEL	0.914 (0.0257)	0.913 (0.0223)	0.908 (0.0187)	0.903 (0.0142)
		ANEL	0.920 (0.0299)	0.915 (0.0233)	0.910 (0.0218)	0.909 (0.0169)
0.95	50	EL	0.939 (0.0622)	0.941 (0.0553)	0.932 (0.0461)	0.886 (0.0325)
		NEL	0.945 (0.0646)	0.943 (0.0568)	0.935 (0.0471)	0.887 (0.0328)
		ANEL	0.955 (0.0726)	0.947 (0.0659)	0.938 (0.0520)	0.912 (0.0378)
	100	EL	0.945 (0.0442)	0.943 (0.0386)	0.931 (0.0327)	0.920 (0.0255)
		NEL	0.949 (0.0457)	0.945 (0.0396)	0.938 (0.0335)	0.930 (0.0258)
		ANEL	0.955 (0.0498)	0.950 (0.0469)	0.946 (0.0385)	0.945 (0.0317)
	150	EL	0.949 (0.0363)	0.948 (0.0322)	0.947 (0.0274)	0.936 (0.0208)
		NEL	0.950 (0.0376)	0.949 (0.0332)	0.949 (0.0277)	0.943 (0.0209)
		ANEL	0.961 (0.0457)	0.953 (0.0373)	0.950 (0.0284)	0.950 (0.0278)
	200	EL	0.951 (0.0313)	0.949 (0.0279)	0.946 (0.0238)	0.945 (0.0187)
		NEL	0.960 (0.0328)	0.957 (0.0288)	0.950 (0.0245)	0.946 (0.0193)
		ANEL	0.965 (0.0348)	0.964 (0.0294)	0.956 (0.0252)	0.953 (0.0213)

Table 3.2: Comparison of coverage probabilities (average lengths) of the confidence intervals with 30% censoring rate, for S1.

Nominal level	Sample size ( $n$ )	Method	$t_0=0.1$	$t_0=0.3$	$t_0=0.5$	$t_0=0.7$
0.90	50	EL	0.872 (0.0554)	0.854 (0.0488)	0.826 (0.0399)	0.761 (0.0236)
		NEL	0.899 (0.0660)	0.878 (0.0562)	0.835 (0.0433)	0.777 (0.0261)
		ANEL	0.900 (0.0667)	0.881 (0.0593)	0.849 (0.0488)	0.784 (0.0351)
	100	EL	0.887 (0.0397)	0.886 (0.0363)	0.883 (0.0316)	0.845 (0.0232)
		NEL	0.906 (0.0487)	0.900 (0.0431)	0.897 (0.0354)	0.855 (0.0249)
		ANEL	0.910 (0.0548)	0.904 (0.0456)	0.900 (0.0384)	0.862 (0.0254)
	150	EL	0.888 (0.0321)	0.887 (0.0294)	0.872 (0.0260)	0.870 (0.0202)
		NEL	0.913 (0.0400)	0.908 (0.0350)	0.899 (0.0290)	0.876 (0.0214)
		ANEL	0.915 (0.0410)	0.912 (0.0375)	0.906 (0.0318)	0.882 (0.0225)
	200	EL	0.909 (0.0283)	0.898 (0.0261)	0.892 (0.0233)	0.889 (0.0183)
		NEL	0.912 (0.0346)	0.908 (0.0308)	0.903 (0.0266)	0.900 (0.0196)
		ANEL	0.919 (0.0391)	0.910 (0.0342)	0.910 (0.0273)	0.903 (0.0201)
0.95	50	EL	0.921 (0.0708)	0.907 (0.0627)	0.869 (0.0510)	0.817 (0.0421)
		NEL	0.937 (0.0818)	0.923 (0.0705)	0.878 (0.0551)	0.839 (0.0432)
		ANEL	0.944 (0.0858)	0.940 (0.0714)	0.905 (0.0651)	0.845 (0.0483)
	100	EL	0.940 (0.0512)	0.939 (0.0473)	0.934 (0.0404)	0.894 (0.0303)
		NEL	0.943 (0.0611)	0.941 (0.0549)	0.936 (0.0452)	0.901 (0.0321)
		ANEL	0.946 (0.0677)	0.948 (0.0557)	0.940 (0.0532)	0.918 (0.0430)
	150	EL	0.945 (0.0393)	0.943 (0.0386)	0.941 (0.0338)	0.925 (0.0264)
		NEL	0.949 (0.0377)	0.940 (0.0374)	0.943 (0.0371)	0.928 (0.0280)
		ANEL	0.952 (0.0438)	0.950 (0.0412)	0.949 (0.0383)	0.929 (0.0282)
	200	EL	0.949 (0.0368)	0.950 (0.0340)	0.945 (0.0302)	0.940 (0.0239)
		NEL	0.956 (0.0435)	0.951 (0.0390)	0.948 (0.0337)	0.943 (0.0256)
		ANEL	0.960 (0.0440)	0.957 (0.0397)	0.953 (0.0342)	0.950 (0.0273)

Table 3.3: Comparison of coverage probabilities (average lengths) of the confidence intervals with 10% censoring rate, for S2.

Nominal level	Sample size ( $n$ )	Method	$t_0=0.5$	$t_0=0.75$	$t_0=1$	$t_0=1.5$
0.90	50	EL	0.821 (0.2058)	0.800 (0.2133)	0.774 (0.2202)	0.656 (0.2240)
		NEL	0.835 (0.2110)	0.809 (0.2176)	0.783 (0.2239)	0.661 (0.2262)
		ANEL	0.840 (0.2116)	0.824 (0.2191)	0.797 (0.2269)	0.671 (0.2266)
	100	EL	0.860 (0.1533)	0.855 (0.1620)	0.835 (0.1726)	0.766 (0.1957)
		NEL	0.871 (0.1581)	0.863 (0.1656)	0.843 (0.1769)	0.772 (0.1986)
		ANEL	0.884 (0.1592)	0.870 (0.1677)	0.867 (0.1770)	0.801 (0.1996)
	150	EL	0.874 (0.1278)	0.866 (0.1367)	0.862 (0.1478)	0.807 (0.1761)
		NEL	0.887 (0.1324)	0.878 (0.1412)	0.869 (0.1519)	0.815 (0.1796)
		ANEL	0.890 (0.1333)	0.888 (0.1438)	0.887 (0.1560)	0.838 (0.1801)
	200	EL	0.881 (0.1100)	0.878 (0.1185)	0.873 (0.1293)	0.829 (0.1588)
		NEL	0.886 (0.1139)	0.880 (0.1226)	0.874 (0.1335)	0.833 (0.1626)
		ANEL	0.899 (0.1147)	0.887 (0.1304)	0.881 (0.1370)	0.854 (0.1634)
0.95	50	EL	0.884 (0.2436)	0.869 (0.2513)	0.803 (0.2617)	0.720 (0.2619)
		NEL	0.891 (0.2495)	0.890 (0.2514)	0.841 (0.2634)	0.723 (0.2635)
		ANEL	0.913 (0.2501)	0.901 (0.2563)	0.897 (0.2639)	0.888 (0.2674)
	100	EL	0.924 (0.1822)	0.909 (0.1922)	0.890 (0.2044)	0.820 (0.2283)
		NEL	0.930 (0.1880)	0.917 (0.1977)	0.900 (0.2094)	0.825 (0.2318)
		ANEL	0.945 (0.1897)	0.922 (0.2005)	0.917 (0.2111)	0.848 (0.2384)
	150	EL	0.934 (0.1535)	0.925 (0.1635)	0.912 (0.1762)	0.869 (0.2074)
		NEL	0.940 (0.1588)	0.930 (0.1687)	0.916 (0.1811)	0.875 (0.2113)
		ANEL	0.949 (0.1603)	0.940 (0.1704)	0.936 (0.1824)	0.888 (0.2122)
	200	EL	0.942 (0.1342)	0.930 (0.1434)	0.925 (0.1561)	0.888 (0.1891)
		NEL	0.945 (0.1382)	0.933 (0.1466)	0.926 (0.1573)	0.895 (0.1943)
		ANEL	0.950 (0.1401)	0.941 (0.1489)	0.938 (0.1599)	0.918 (0.1949)

Table 3.4: Comparison of coverage probabilities (average lengths) of the confidence intervals with 30% censoring rate, for S2.

Nominal level	Sample size ( $n$ )	Method	$t_0=0.5$	$t_0=0.75$	$t_0=1$	$t_0=1.5$
0.90	50	EL	0.780 (0.2098)	0.745 (0.2162)	0.711 (0.2210)	0.564 (0.2252)
		NEL	0.805 (0.2241)	0.768 (0.2287)	0.729 (0.2305)	0.579 (0.2336)
		ANEL	0.828 (0.2319)	0.784 (0.2349)	0.732 (0.2355)	0.621 (0.2363)
	100	EL	0.811 (0.1772)	0.795 (0.1866)	0.765 (0.1961)	0.668 (0.2039)
		NEL	0.844 (0.1947)	0.823 (0.2022)	0.784 (0.2093)	0.684 (0.2118)
		ANEL	0.859 (0.1997)	0.844 (0.2066)	0.809 (0.2099)	0.711 (0.2154)
	150	EL	0.826 (0.1499)	0.812 (0.1594)	0.792 (0.1705)	0.709 (0.1919)
		NEL	0.865 (0.1664)	0.845 (0.1753)	0.818 (0.1849)	0.725 (0.2018)
		ANEL	0.884 (0.1671)	0.863 (0.1762)	0.827 (0.1896)	0.734 (0.2026)
	200	EL	0.851 (0.1146)	0.839 (0.1226)	0.822 (0.1334)	0.748 (0.1623)
		NEL	0.864 (0.1314)	0.854 (0.1414)	0.849 (0.1532)	0.806 (0.1800)
		ANEL	0.879 (0.1316)	0.871 (0.1420)	0.868 (0.1540)	0.835 (0.1809)
0.95	50	EL	0.840 (0.2478)	0.812 (0.2547)	0.775 (0.2624)	0.627 (0.2641)
		NEL	0.854 (0.2641)	0.823 (0.2683)	0.789 (0.2694)	0.636 (0.2700)
		ANEL	0.860 (0.2644)	0.846 (0.2691)	0.798 (0.2708)	0.651 (0.2715)
	100	EL	0.874 (0.2093)	0.852 (0.2194)	0.826 (0.2296)	0.725 (0.2371)
		NEL	0.893 (0.2300)	0.875 (0.2375)	0.846 (0.2453)	0.737 (0.2466)
		ANEL	0.904 (0.2308)	0.896 (0.2381)	0.886 (0.2460)	0.771 (0.2473)
	150	EL	0.886 (0.1774)	0.875 (0.1886)	0.853 (0.2011)	0.763 (0.2242)
		NEL	0.911 (0.1974)	0.901 (0.2072)	0.879 (0.2181)	0.779 (0.2348)
		ANEL	0.920 (0.1983)	0.919 (0.2081)	0.887 (0.2199)	0.787 (0.2354)
	200	EL	0.906 (0.1572)	0.896 (0.1684)	0.881 (0.1817)	0.815 (0.2111)
		NEL	0.937 (0.1659)	0.924 (0.1732)	0.905 (0.1819)	0.827 (0.2114)
		ANEL	0.944 (0.1668)	0.929 (0.1734)	0.911 (0.1825)	0.851 (0.2124)



Table 3.5: Comparison of coverage probabilities (average lengths) of the confidence intervals with 10% censoring rate, for S3.

Nominal level	Sample size ( $n$ )	Method	$t_0=1$	$t_0=2$	$t_0=3$	$t_0=4$
0.90	50	EL	0.732 (1.1422)	0.705 (1.1974)	0.689 (1.2314)	0.662 (1.2855)
		NEL	0.748 (1.1644)	0.724 (1.2281)	0.706 (1.2892)	0.678 (1.3137)
		ANEL	0.759 (1.1661)	0.730 (1.2290)	0.710 (1.2899)	0.699 (1.3185)
	100	EL	0.800 (0.9313)	0.787 (1.0316)	0.766 (1.1533)	0.749 (1.2622)
		NEL	0.821 (0.9480)	0.810 (1.0681)	0.792 (1.1640)	0.769 (1.2873)
		ANEL	0.847 (0.9482)	0.825 (1.0688)	0.799 (1.1652)	0.788 (1.2880)
	150	EL	0.840 (0.7501)	0.825 (0.8540)	0.810 (0.9947)	0.790 (1.1455)
		NEL	0.864 (0.8120)	0.753 (0.9323)	0.737 (1.0738)	0.822 (1.2022)
		ANEL	0.870 (0.8131)	0.760 (0.9330)	0.745 (1.0749)	0.829 (1.2033)
	200	EL	0.846 (0.6048)	0.837 (0.7260)	0.824 (0.8838)	0.813 (1.0377)
		NEL	0.872 (0.6270)	0.866 (0.8198)	0.851 (0.9498)	0.840 (1.0921)
		ANEL	0.875 (0.6282)	0.871 (0.8204)	0.858 (0.9504)	0.848 (0.0924)
0.95	50	EL	0.803 (1.9211)	0.785 (1.9906)	0.758 (2.0580)	0.729 (2.1100)
		NEL	0.815 (1.9418)	0.793 (2.0040)	0.769 (2.0601)	0.744 (2.1201)
		ANEL	0.831 (1.9422)	0.810 (2.0047)	0.800 (2.0613)	0.769 (2.1211)
	100	EL	0.878 (1.7148)	0.861 (1.8217)	0.843 (1.9838)	0.819 (2.0891)
		NEL	0.889 (1.7474)	0.873 (1.8769)	0.858 (1.9924)	0.835 (2.1199)
		ANEL	0.905 (1.7484)	0.893 (1.8771)	0.866 (1.9937)	0.849 (2.1206)
	150	EL	0.931 (1.5472)	0.896 (1.5546)	0.884 (1.7253)	0.870 (1.8916)
		NEL	0.938 (1.5512)	0.922 (1.7254)	0.904 (1.9072)	0.888 (2.0749)
		ANEL	0.940 (1.5518)	0.929 (1.7260)	0.919 (1.9085)	0.891 (2.0754)
	200	EL	0.946 (1.3881)	0.908 (1.3990)	0.895 (1.5940)	0.882 (1.7951)
		NEL	0.948 (1.4179)	0.927 (1.5704)	0.920 (1.7487)	0.909 (1.9571)
		ANEL	0.949 (1.4189)	0.934 (1.5714)	0.927 (1.7491)	0.912 (1.9579)

Table 3.6: Comparison of coverage probabilities (average lengths) of the confidence intervals with 30% censoring rate, for S3.

Nominal level	Sample size ( $n$ )	Method	$t_0=1$	$t_0=2$	$t_0=3$	$t_0=4$
0.90	50	EL	0.548 (2.1983)	0.519 (2.2227)	0.500 (2.2553)	0.465 (2.2701)
		NEL	0.564 (2.2753)	0.536 (2.3183)	0.511 (2.3813)	0.482 (2.3977)
		ANEL	0.575 (2.2761)	0.549 (2.3191)	0.530 (2.3819)	0.492 (2.3982)
	100	EL	0.645 (2.0887)	0.616 (2.1036)	0.602 (2.1881)	0.571 (2.2522)
		NEL	0.662 (2.1340)	0.640 (2.1557)	0.616 (2.2487)	0.590 (2.2599)
		ANEL	0.669 (2.1343)	0.651 (2.1563)	0.626 (2.2491)	0.605 (2.2604)
	150	EL	0.684 (2.0045)	0.662 (2.0498)	0.640 (2.1111)	0.620 (2.2366)
		NEL	0.704 (2.0481)	0.682 (2.0992)	0.662 (2.2025)	0.634 (2.2449)
		ANEL	0.718 (2.0488)	0.693 (2.0999)	0.675 (2.2040)	0.660 (2.2452)
	200	EL	0.685 (1.9516)	0.662 (2.0103)	0.645 (2.0610)	0.621 (2.0695)
		NEL	0.714 (2.0202)	0.695 (2.0755)	0.669 (2.1197)	0.641 (2.1425)
		ANEL	0.730 (2.0215)	0.712 (2.0759)	0.679 (2.1206)	0.666 (2.1455)
0.95	50	EL	0.603 (2.8973)	0.574 (2.9305)	0.541 (3.0239)	0.508 (3.1289)
		NEL	0.628 (2.9707)	0.594 (3.0978)	0.580 (3.1068)	0.523 (3.2122)
		ANEL	0.641 (2.9717)	0.620 (3.0982)	0.600 (3.1070)	0.534 (3.2127)
	100	EL	0.719 (2.7927)	0.696 (2.8253)	0.670 (2.9128)	0.637 (3.0770)
		NEL	0.729 (2.8671)	0.699 (2.8797)	0.679 (2.9623)	0.648 (3.1506)
		ANEL	0.736 (2.8679)	0.702 (2.8803)	0.698 (2.9628)	0.681 (3.1517)
	150	EL	0.758 (2.6307)	0.737 (2.6956)	0.715 (2.7443)	0.686 (2.7894)
		NEL	0.765 (2.6726)	0.747 (2.7089)	0.719 (2.7614)	0.694 (2.8665)
		ANEL	0.784 (2.6731)	0.750 (2.7098)	0.723 (2.7630)	0.724 (2.8677)
	200	EL	0.763 (2.5301)	0.742 (2.6179)	0.716 (2.6913)	0.649 (2.7790)
		NEL	0.801 (2.6409)	0.760 (2.7024)	0.740 (2.7548)	0.710 (2.8003)
		ANEL	0.831 (2.6421)	0.782 (2.7051)	0.754 (2.7559)	0.751 (2.8123)

Table 3.7: Comparison of coverage probabilities (average length) of the 95% confidence intervals with 10% censoring rate, for S1.

Nominal level	$n$	Method	$t_0 = 0.1$	$t_0 = 0.3$	$t_0 = 0.5$	$t_0 = 0.7$
0.95	500	EL	0.9503 (0.0191)	0.9503(0.0176)	0.9501 (0.0169)	0.9500 (0.0138)
		NEL	0.9552 (0.0192)	0.9552(0.0180)	0.9541 (0.0165)	0.9539 (0.0122)
	1000	EL	0.9489 (0.0131)	0.9487(0.0130)	0.9493 (0.0115)	0.9484 (0.0113)
		NEL	0.9501 (0.0135)	0.9500(0.0131)	0.9500 (0.0110)	0.9499 (0.0106)
	5000	EL	0.9492 (0.0051)	0.9492(0.0047)	0.9491 (0.0037)	0.9485 (0.0020)
		NEL	0.9500 (0.0053)	0.9501(0.0049)	0.9500 (0.0041)	0.9499 (0.0027)

slightly wider than the EL-based confidence intervals.

- 4) The coverage probability is negatively affected by the skewness of the distribution, the censorship, and the time  $t_0$ . The more skewed the distribution is and the greater the time  $t_0$  is and the higher the censorship is, the lower the coverage probability is. That explains why the coverage probabilities in case S3 with 30% censoring are much lower than their nominal values.
- 5) For small samples, and in some situations (see S2 and S3), both methods EL and NEL have low coverage probabilities. The ANEL uniformly improves those coverages by extending the confidence intervals.
- 6) For the uniform distribution (S1), we observe a little over-coverage for the sample size  $n = 150$  or  $n = 200$  and mainly for  $t_0 = 0.1$ . However, when sample sizes increase to  $n = 500, 1000$ , and  $5000$ , the coverage probabilities decrease and become closer to the given nominal level (see Table 3.7). Zheng, Shen, and He (2014) encountered these findings as well. Though this behavior apparently is not due to random variation, and we could not find a theoretical justification, we notice that the coverage probabilities become more stable when the sample size increases considerably.

### 3.4 Real Applications

In this section, we apply the proposed method to estimate the mean residual lifetime for the Channing House data. A complete description of this data set can be found in Klein and Moeschberger (1997). Channing House is a retirement center located in Palo Alto, California. The data set contains the sex, the ages at entry, the ages at death (or leaving the center) and censoring indicators of 462 retirees, who are composed of 97 men and 365 women and were collected from January 1964 to July 1975 (Hyde, 1980). During the study, 46 men and 130 women died at Channing House. The individuals who left Channing House or were still in the center at the end of the study were censored. Because an individual must survive to a sufficient age to enter the retirement center, the data are left-truncated and

right-censored. The entry age is considered as the left-truncation time. A sub-sample of this data (448 people), which includes the individuals whose ages at entry are more than 65.5 years (786 months), can be seen as length-biased and right-censored data (Chen and Zhou, 2012). The stationary assumption for this sub-sample has been checked by the methods in Addona and Wolfson (2006) and Asgharian, Borgan, Gill, and Keiding (2006). The 90% confidence intervals for the mean residual life function of people in the center based on the length-biased sub-sample are calculated at selected ages 70, 75, 80, 85, 90 and 95 and summarized in Table 3.8.

Table 3.8: 90% confidence intervals for the MRL at the selected ages for the Channing house data.

age (years)	70	75	80	85	90	95
EL	(10.08, 11.06)	(8.03, 9.05)	(6.18, 7.26)	(4.77, 5.87)	(3.48, 4.43)	(2.06, 2.57)
NEL	(9.62, 10.85)	(7.54, 8.83)	(5.67, 7.03)	(4.24, 5.64)	(2.97, 4.24)	(1.62, 2.39)
ANEL	(9.52, 11.15)	(7.46, 9.11)	(5.61, 7.27)	(4.13, 5.80)	(2.85, 4.29)	(1.57, 2.39)

Table 3.9: 90% confidence intervals for the MRL at the selected ages for men versus women for the Channing house data.

age (years)	Men			Women		
	EL	NEL	ANEL	EL	NEL	ANEL
70	(9.14, 10.41)	(8.09, 9.97)	(8.04, 10.24)	(10.13, 11.27)	(9.65, 11.04)	(9.55, 11.32)
75	(7.07, 8.33)	(5.99, 7.89)	(5.30, 8.11)	(8.07, 9.25)	(7.56, 9.01)	(7.40, 9.27)
80	(5.12, 6.36)	(3.93, 5.91)	(3.36, 6.08)	(6.24, 7.46)	(5.70, 7.21)	(5.65, 7.43)
85	(3.45, 4.60)	(2.01, 4.14)	(1.74, 4.22)	(4.86, 6.06)	(4.32, 5.83)	(4.22, 5.96)
90	(1.96, 2.78)	(0, 2.37)	(0, 2.37)	(3.57, 4.54)	(3.06, 4.36)	(2.96, 4.39)
95	—	—	—	(2.04, 2.56)	(1.60, 2.39)	(1.58, 2.39)

We notice that, as the age increases, the MRL decreases in general. Table 3.8 confirmed the results of the simulation study: NEL and ANEL confidence intervals for the mean residual life at selected ages are slightly wider than EL confidence intervals.

Finally, in Table 3.9, we compared different confidence intervals for men versus women in the center and reached the well-known conclusion that women have greater MRL than men, meaning, tend to live longer than men at the same given age.

### 3.5 Conclusions

In this chapter, we have considered new empirical likelihood confidence intervals for the mean residual life function with length-biased and right-censored data based on the estimating equation in Liang et al. (2016). We have shown that the NEL log-likelihood ratio converges to a chi-squared distribution instead of the scaled chi-squared from Liang et al. (2016). We have also proposed the adjusted NEL for the MRL. A confidence interval is then constructed for the MRL at time  $t_0$  by using the proposed methods and compared with the empirical likelihood-based (EL) method via simulations. Not only do the proposed confidence intervals tend to the nominal level when the sample size increases for all approaches, but also, the NEL and ANEL outperform the EL method in terms of coverage probability at the cost of having a wider average length of confidence intervals. It is also easy to implement the proposed method using existing R packages. Finally, a real application is given to illustrate the performance of the proposed EL methods.

## CHAPTER 4

### NOVEL EMPIRICAL LIKELIHOOD INFERENCE FOR THE MEAN DIFFERENCE WITH RIGHT-CENSORED DATA

#### 4.1 Background

Examples of comparing two means with two independent or dependent samples are numerous. It is the case in a lot of two-sample problems. For example, in a case-control study, epidemiologists compare two treatments (one treatment group can be a control group). In clinical trials, the effects of two drugs (one drug can be a placebo) on patients are compared to determine how these drugs affect them. In the reliability, the mean lifetimes of electric components obtained using different procedures or from various providers are analyzed, etc.

Parametric methods such as two-sample  $Z$ -test for large samples or two-sample Student's  $t$ -test for small samples are used when the underlying distributions of the samples are assumed to be normal. The likelihood ratio test also is used for hypothesis testing. In practice, the sampling distributions are unknown, and these methods need to be adjusted. Nonparametric and semiparametric methods are then used, and one method that has proven to give good results in these cases is the EL approach.

For two uncensored samples problems, Qin (1994) developed a semiparametric EL model (maximum likelihood for one sample and EL for the other sample ) for the mean difference. Jing (1995) and later, Liu et al. (2008) applied the EL method and showed that it is Bartlett-correctable. For case-control data, Qin (1998) studied semiparametric method inference, and Zhang (2000) found EL based confidence intervals for the treatment effect with auxiliary information. A weighted version of the two-sample EL for the mean is proposed by Wu and Yan (2012). Often, these methods can be extended to parameters different from the sample mean. In this way, Claeskens et al. (2003) used EL to compare two distributions and find their confidence regions. Zhou and Jing (2003) extended the EL method to the differ-

ence of quantiles problem, and compare it with the normal approximation method. Liu and Zhao (2012) used the semi-parametric EL approach to construct confidence intervals for the ROC curves of two populations with missing data. The jackknife empirical likelihood (JEL) method for the difference of two correlated continuous-scale ROC curves, the difference of two quantiles, and the one-sample difference of quantiles are proposed by Yang and Zhao (2013, 2016, 2018) to avoid estimating link variables associated with the EL method. An and Zhao (2017) went further and applied the JEL for the difference of two correlated volumes under ROC surfaces. This technique is used to compare two treatments for discrimination of three-class classification data. Recently, Liang, Dai, and He (2019) developed the mean empirical likelihood (MEL), based on the pairwise-means of the data. Their EL statistic is a weighted sum of chi-squared random variables.

Some work has also been done for censored samples. Wang and Wang (2001) introduced inference in the two-sample random censorship model by the synthetic data approach. They had to deal with a scale parameter, which made their method complicated. Zhou and Liang (2005) applied the EL-based semiparametric inference method to the treatment effect. Some extensions are remarkable for censored samples. In a series of publications, McKeague and Zhao (2002, 2005, 2006) established the foundation of comparing two distributions or two survival functions by constructing confidence bands for their difference or ratio. Using smoothed EL methods, Zhao and Zhao (2011) constructed confidence intervals for the ratio and difference of two hazard functions. The EL is also used by Zhao et al. (2016) to estimate odds ratios of survival functions. To our knowledge, no paper used the i.i.d. representation approach (He et al., 2016) for the two-sample inference with right-censored data.

In the present chapter, we use this i.i.d. representation technique and propose an EL inference procedure for the mean difference for right-censored data. This method is denoted IID-EL. We prove that, under some regularity conditions, the limiting distribution of the empirical log-likelihood ratio is a standard chi-squared. This method allows for avoiding estimating the scale parameter from Wang and Wang (2001). We can construct an IID-EL-based confidence interval for the mean difference using the established asymptotic property.

Simulation studies show that the proposed IID-EL-based intervals have better coverage accuracy than the scaled EL intervals from Wang and Wang (2001).

The rest of the chapter is organized as follows. In Section 4.2, we show the proposed method and state the main asymptotic results. In Section 4.3, a simulation study is carried out to compare the performance of proposed IID-EL method with the EL method from Wang and Wang (2001) in terms of coverage probability and the average length of the confidence interval. In Section 4.4, an application to the PBC data set is provided. Section 4.5 contains the conclusions. The proofs are relegated in the Appendix C.

## 4.2 Main Results

For all the EL methods described in the following subsections, we consider the two-sample random censorship model. Let  $\{X_1^0, X_2^0, \dots, X_m^0\}$  and  $\{Y_1^0, Y_2^0, \dots, Y_n^0\}$  be i.i.d. positive random variables with the CDF  $F$  and  $G$  from two different populations  $X^0$  and  $Y^0$ , respectively. These two samples are randomly censored by the two sets of random variables  $\{U_1, U_2, \dots, U_m\}$  and  $\{V_1, V_2, \dots, V_n\}$  having CDF  $K$  and  $Q$ , respectively.  $X_i^0$  and  $Y_j^0$  are not directly observable but, instead we observe  $(X_i, \delta_i)$  and  $(Y_j, \eta_j)$  for  $i = 1, 2, \dots, m$  and  $j = 1, 2, \dots, n$ , where

$$X_i = \min(X_i^0, U_i), \quad \delta_i = I(X_i^0 \leq U_i), \quad i = 1, 2, \dots, m,$$

$$Y_j = \min(Y_j^0, V_j), \quad \eta_j = I(Y_j^0 \leq V_j), \quad j = 1, 2, \dots, n.$$

In this model,  $F$ ,  $G$ ,  $K$ , and  $Q$  are assumed continuous and unknown. All the variables  $X_i^0$ ,  $U_i$ ,  $Y_j^0$ , and  $V_j$  are supposed to be mutually independent, for  $i = 1, 2, \dots, m$  and  $j = 1, 2, \dots, n$ , and  $I(\cdot)$  is the indicator function.

### 4.2.1 I.i.d. empirical likelihood (IID-EL)

This subsection describes how the i.i.d. empirical likelihood method, denoted IID-EL, is established. Let  $\theta_1$  and  $\theta_2$  be the respective means of the two populations  $X^0$  and  $Y^0$ . We



are interested in the inference on the difference  $\theta = \theta_1 - \theta_2$ . Let  $\theta = \theta(F, G) = EX^0 - EY^0$ . Then, by the inverse probability weighting theory, we have

$$\theta(F, G) = E \frac{\delta X}{1 - K(X-)} - E \frac{\eta Y}{1 - Q(Y-)}. \quad (4.1)$$

To apply the EL, we will replace the unknown  $K$  and  $Q$  by their Kaplan-Meier estimators  $\widehat{K}_m$  and  $\widehat{Q}_n$ ,

$$1 - \widehat{K}_m(t) = \prod_{i=1}^m \left[ \frac{m-i}{m-i+1} \right]^{I(X_{(i)} \leq t, \delta_{(i)}=0)}$$

and

$$1 - \widehat{Q}_n(t) = \prod_{j=1}^n \left[ \frac{n-j}{n-j+1} \right]^{I(Y_{(j)} \leq t, \eta_{(j)}=0)},$$

where  $\{X_{(1)}, X_{(2)}, \dots, X_{(m)}\}$  (respectively  $\{Y_{(1)}, Y_{(2)}, \dots, Y_{(n)}\}$ ) are the ordered values of the sample  $X$  ( respectively the sample  $Y$ ) and  $\{\delta_{(1)}, \delta_{(2)}, \dots, \delta_{(m)}\}$  (respectively  $\{\eta_{(1)}, \eta_{(2)}, \dots, \eta_{(n)}\}$ ) the corresponding values of  $\delta$  associated to  $X_{(i)}$ (respectively  $\eta$  associated to  $Y_{(j)}$ ). Then, one will test

$$E \frac{\delta X}{1 - \widehat{K}_m(X-)} - E \frac{\eta Y}{1 - \widehat{Q}_n(Y-)} \approx \theta. \quad (4.2)$$

Based on this result, Wang and Wang (2001) derived EL confidence intervals for the mean difference  $\theta$ . However, their estimated EL ratio involves the estimated censoring distributions  $\widehat{K}_m$  and  $\widehat{Q}_n$  and converges to a scaled  $\chi_1^2$  distribution. The scale parameter is a function of some unknown asymptotic variances from both samples, which need to be estimated. The results of this EL are affected by the variance estimators. For example, the plug-in estimator is not stable, in case of the right censoring, and it leads to the low coverage probability. On the one hand, Wang and Wang (2001) used the modified jackknife estimator of the asymptotic variance proposed by Stute (1996) to achieve their results (see Wang and Wang, 2001), on the other hand, the method is time-consuming. One of the advantages of the EL method over the non-EL existing methods is that the EL does not need the variance estimation, but the presence of the scale parameter removes this advantage from the EL. Our approach tries to restore this primary advantage to the EL. As one notices, the relation (2.2) involves

the Kaplan-Meier weights  $\left(1 - \widehat{K}_m(X_{i-})\right)^{-1}$  and  $\left(1 - \widehat{Q}_n(Y_{j-})\right)^{-1}$ , for  $i = 1, 2, \dots, m$  and  $j = 1, 2, \dots, n$ . The i.i.d. representations of Kaplan-Meier estimators exist in the literature (see Yang, 1997), and they will be used to obtain our main result. The main idea is to write the weights as the sum of i.i.d. random variables plus a remainder.

By replacing  $\left(1 - \widehat{K}_m(X_{i-})\right)^{-1}$  and  $\left(1 - \widehat{Q}_n(Y_{j-})\right)^{-1}$  by their i.i.d. representations from He et al. (2016) (see Equation (3.2)), we let

$$V_i = \frac{X_i \delta_i}{1 - K(X_{i-})} + \frac{1 - \delta_i}{\overline{H}(X_{i-})} \varphi(X_i) - \int_0^\infty \varphi(s) \frac{I(X_i \geq s)}{H^2(s-)} dH^0(s), \quad (4.3)$$

where  $H(x) = P(X_1 \leq x)$ ,  $H^0(x) = P(X_1 > x, \delta_1 = 0)$  and  $\varphi(x) = \int_x^\infty sdF(s)$ ,

$$W_j = \frac{Y_j \eta_j}{1 - Q(Y_{j-})} + \frac{1 - \eta_j}{\overline{L}(Y_{j-})} \psi(Y_j) - \int_0^\infty \psi(s) \frac{I(Y_j \geq s)}{L^2(s-)} dL^0(s), \quad (4.4)$$

where  $L(y) = P(Y_1 \leq y)$ ,  $L^0(y) = P(Y_1 > y, \eta_1 = 0)$  and  $\psi(y) = \int_y^\infty sdG(s)$  and for any functional  $A$ ,  $\overline{A} = 1 - A$ .

$V_i$  and  $W_j$  are i.i.d. random variables with means  $EV_1 = \theta_1$  and  $EW_1 = \theta_2$  for  $i = 1, \dots, m$  and  $j = 1, 2, \dots, n$ . At this point,  $\theta(F, G) = EV - EW$  and the usual EL (see Owen, 1990) to test whether  $\theta$  is equal to a given value or to construct confidence interval for  $\theta$  can be applied. But  $V_i$  and  $W_j$  are not observable, due to the presence of the unknown  $K, H, H^0, F, Q, L, L^0$ , and  $G$ . To define an estimated EL ratio,  $F, G, K$ , and  $Q$  are replaced by their Kaplan-Meier estimators  $\widehat{F}_n, \widehat{G}_n, \widehat{K}_n$ , and  $\widehat{Q}_n$ , respectively, with

$$\widehat{F}_m(t) = 1 - \prod_{i=1}^m \left[ \frac{m-i}{m-i+1} \right]^{I(X_{(i)} \leq t, \delta_{(i)}=1)},$$

$$\widehat{G}_n(t) = 1 - \prod_{j=1}^n \left[ \frac{n-j}{n-j+1} \right]^{I(Y_{(j)} \leq t, \eta_{(j)}=1)},$$

$H, H^0, L,$  and  $L^0$  by their empirical counterparts given by

$$H_m(x) = \frac{1}{m} \sum_{k=1}^m I(X_k \leq x), L_n(y) = \frac{1}{n} \sum_{k=1}^n I(Y_k \leq y),$$

$$H_m^0(x) = \frac{1}{m} \sum_{k=1}^m I(X_k \leq x, \delta_k = 0), L_n^0(y) = \frac{1}{n} \sum_{k=1}^n I(Y_k \leq y, \eta_k = 0),$$

$\varphi(x)$  by  $\varphi_m(x) = \int_x^\infty s d\widehat{F}_m(s)$  and  $\psi(y)$  by  $\psi_n(y) = \int_y^\infty s d\widehat{G}_n(s)$ .

We obtain

$$V_{mi} = \frac{X_i \delta_i}{1 - \widehat{K}_m(X_{i-})} + \frac{1 - \delta_i}{\widehat{H}_m(X_{i-})} \varphi_m(X_i) - \int_0^\infty \varphi_m(s) \frac{I(X_i \geq s)}{\widehat{H}_m^2(s-)} dH_m^0(s) \quad (4.5)$$

and

$$W_{nj} = \frac{Y_j \eta_j}{1 - \widehat{Q}_n(Y_{j-})} + \frac{1 - \eta_j}{\widehat{L}_n(Y_{j-})} \psi_n(Y_j) - \int_0^\infty \psi_n(s) \frac{I(Y_j \geq s)}{\widehat{L}_n^2(s-)} dL_n^0(s). \quad (4.6)$$

One fundamental property of  $V_{mi}$  and  $W_{nj}$  is stated as follows.

**Theorem 4.2.1.** *Let  $V_{mi}$  and  $W_{nj}$  be defined as in equations (4.5) and (4.6). Under the conditions that  $\int_0^\infty s^2 / \overline{K}(s) dF(s) < \infty$ , and  $\int_0^\infty s^2 / \overline{Q}(s) dG(s) < \infty$ , as  $m \rightarrow \infty, n \rightarrow \infty$ , it holds that*

$$\frac{1}{\sqrt{m}} \sum_{i=1}^m (V_{mi} - \theta_1) - \frac{1}{\sqrt{m}} \sum_{i=1}^m (V_i - \theta_1) = o_p(1)$$

and

$$\frac{1}{\sqrt{n}} \sum_{j=1}^n (W_{nj} - \theta_2) - \frac{1}{\sqrt{n}} \sum_{j=1}^n (W_j - \theta_2) = o_p(1).$$

Theorem 4.2.1 is just an adaptation of Theorem 3.1 in He et al. (2016) with the  $i$ -th influence function  $V_i - \theta_1$  for given  $\theta_1$  (respectively  $j$ -th influence function  $W_j - \theta_2$  for given  $\theta_2$ ). Hence the reader can refer to this paper for the proof. Zheng, Zhao, and Yu (2012) proposed the influence function-based EL method when the estimating functions contain nuisance parameters. They proved that under some mild conditions, the log-EL ratio statistics limiting distributions are chi-squared. The same idea also can be found in Sun et al. (2009) for confidence regions for the time-dependent regression coefficients in

Cox models; in Zhao and Huang (2007) and Wu et al. (2015) for interval estimate of the parameter in AFT models; in Huang and Zhao (2018) for inference on the bivariate survival function, etc. Theorem 4.2.1 is useful to prove Lemma C.1 in the Appendix C, which contains the relations leading to our main result.

**Remark 4.2.1.** *The technique of using influence functions is also known under the denomination i.i.d. representation as  $\sum_{i=1}^m (V_{mi} - \theta_1)$  asymptotically can be written as a partial sum of  $m$  i.i.d. influence functions  $V_i - \theta_1$ , for example.*

**Remark 4.2.2.** *Although both  $V_{mi}$ 's and  $W_{nj}$ 's are not independent, they can be used to construct the EL ratio and obtain the standard  $\chi_1^2$  distribution asymptotically. The main reason (see the above mentioned Lemma C.1) is that the asymptotic variance of  $1/\sqrt{m} \sum_{i=1}^m (V_{mi} - \theta_1)$  is the limit of  $1/m \sum_{i=1}^m (V_{mi} - \theta_1)^2$ , which is  $\sigma_1^2 = \text{var}(V_i)$ . We have the similar result for  $W_{nj}$ .*

Let  $\mathbf{p}_1 = (p_{11}, p_{12}, \dots, p_{1m})'$  and  $\mathbf{p}_2 = (p_{21}, p_{22}, \dots, p_{2n})'$  be two probability vectors over the two samples  $V_m$  and  $W_n$ , respectively, with

$$\sum_{i=1}^m p_{1i} = 1, p_{1i} > 0, \sum_{j=1}^n p_{2j} = 1, p_{2j} > 0.$$

To find the IID-EL ratio for  $\theta$ , denoted  $r(\theta)$ , based on  $V_{mi}$ 's and  $W_{nj}$ 's, we consider one of the parameters ( $\theta_1$  for example) as a nuisance parameter, and define the estimated IID-EL ratio at  $(\theta_1, \theta)$  as

$$R(\theta_1, \theta) = \sup \left\{ \prod_{i=1}^m (m p_{1i}) \prod_{j=1}^n (n p_{2j}) : \sum_{i=1}^m p_{1i} V_{mi} = \theta_1; \sum_{j=1}^n p_{2j} W_{nj} = \theta_1 - \theta \right\}.$$

Note that  $\theta_1$  will eventually need to be profiled out. The estimated IID-EL log-likelihood at  $(\theta_1, \theta)$  (see Owen 1988, 1990) is

$$l(p_1, p_2) = \sum_{i=1}^m \log(p_{1i}) + \sum_{j=1}^n \log(p_{2j}),$$

and the estimated IID-EL log-likelihood ratio is then

$$\log R(\theta_1, \theta) = \sum_{i=1}^m \log(m\hat{p}_{1i}(\theta_1)) + \sum_{j=1}^n \log(n\hat{p}_{2j}(\theta_1, \theta)),$$

where  $\hat{p}_{1i}(\theta_1)$  and  $\hat{p}_{2j}(\theta_1, \theta)$  maximize  $l(p_1, p_2)$  with the constraints

$$\begin{cases} \sum_{i=1}^m p_{1i} = 1, \sum_{j=1}^n p_{2j} = 1 \\ \sum_{i=1}^m p_{1i} V_{mi} = \theta_1 \\ \sum_{j=1}^n p_{2j} W_{nj} = \theta_1 - \theta. \end{cases}$$

Using Lagrange multipliers, it is easy to show that  $\hat{p}_{1i}(\theta_1)$  and  $\hat{p}_{2j}(\theta_1, \theta)$  which maximize  $l(p_1, p_2)$  are given by

$$\hat{p}_{1i} = \frac{1}{m \{1 + \lambda_1(V_{mi} - \theta_1)\}}, \quad i = 1, \dots, m$$

and

$$\hat{p}_{2j} = \frac{1}{n \{1 + \lambda_2(W_{nj} - \theta_1 + \theta)\}}, \quad j = 1, \dots, n,$$

where the Lagrange multipliers  $\lambda_1$  and  $\lambda_2$  are solutions to the following equations

$$\frac{1}{m} \sum_{i=1}^m \frac{V_{mi} - \theta_1}{1 + \lambda_1(V_{mi} - \theta_1)} = 0 \quad (4.7)$$

and

$$\frac{1}{n} \sum_{j=1}^n \frac{W_{nj} - \theta_1 + \theta}{1 + \lambda_2(W_{nj} - \theta_1 + \theta)} = 0. \quad (4.8)$$

The log-EL ratio becomes

$$\log R(\theta_1, \theta) = - \sum_{i=1}^m \log \{1 + \lambda_1(V_{mi} - \theta_1)\} - \sum_{j=1}^n \log \{1 + \lambda_2(W_{nj} - \theta_1 + \theta)\}. \quad (4.9)$$

To derive the IID-EL ratio function for  $\theta$ , we first need to find the  $\widehat{\theta}_1$ , which maximizes  $\log R(\theta_1, \theta)$  for  $\theta$  fixed. Theorem 1 from Qin and Zhao (2000), ensures the existence of  $\widehat{\theta}_1$  such that  $\widehat{\theta}_1$  is a consistent estimate of  $\theta_1$ . In addition,  $R(\theta_1, \theta)$  attains its local maximum value at  $\widehat{\theta}_1$  and

$$\sqrt{m}(\widehat{\theta}_1 - \theta_1) \xrightarrow{D} N\left(0, \frac{\beta_1\beta_2}{\beta_2 + k\beta_1}\right), \quad (4.10)$$

where  $\beta_1 = E(V_m - \theta_1)^2$ ,  $\beta_2 = E(W_n - \theta_1 + \theta)^2$  and  $m/n \rightarrow k$  ( $k > 0$ ) as  $m, n \rightarrow \infty$  and  $\xrightarrow{D}$  means converges in distribution.

Plugging  $\widehat{\theta}_1$  into the log-likelihood ratio function, we have  $\log r(\theta) \triangleq \log R(\widehat{\theta}_1, \theta)$  and the following theorem is established.

**Theorem 4.2.2.** *Let  $\theta_0$  be the true value of  $\theta$ . Let  $s = m + n$ . Assume the conditions of Theorem 4.2.1 hold and  $m/s = \delta \rightarrow \delta_0 \in (0, 1)$  as  $s \rightarrow \infty$ . Then  $-2\log r(\theta_0) \xrightarrow{D} \chi_1^2$ , as  $s \rightarrow \infty$ ,*

where  $\chi_1^2$  is a standard chi-squared random variable with one degree of freedom.

Thus, using Theorem 4.2.2, an asymptotic  $100(1 - \alpha)\%$  IID-EL confidence interval for  $\theta$  is given by

$$I = \{\theta : -2\log r(\theta) \leq \chi_1^2(\alpha)\},$$

where  $\chi_1^2(\alpha)$  is the upper  $\alpha$ -quantile of the distribution of  $\chi_1^2$ .

#### 4.2.2 I.i.d. adjusted empirical likelihood (IID-AEL)

The adjusted empirical likelihood (AEL) introduced by Chen, Variyath, and Abraham (2008) aims mainly to ensure that the EL ratio is always well-defined by adding a pseudo point to the original dataset so that the convex hull of the augmented dataset contains the true mean value. This approach often can solve the under-coverage problem encountered by the EL method for small samples. We apply this technique to the IID-EL and then obtained the IID-AEL. For this purpose, we add a pseudo-data to each sample

$$V_{mm+1} - \theta_1 = -\frac{a_m}{m} \sum_{i=1}^m (V_{mi} - \theta_1); W_{nn+1} - \theta_1 + \theta = -\frac{a_n}{n} \sum_{j=1}^n (W_{nj} - \theta_1 + \theta),$$

where  $a_m = \max(1, \log(m)/2)$  and  $a_n = \max(1, \log(n)/2)$ .

Adopting the same methodology as in the subsection 4.2.1, we define the estimated IID-AEL ratio at  $(\theta_1, \theta)$  as

$$R^A(\theta_1, \theta) = \sup \left\{ \prod_{i=1}^{m+1} (m+1)p_{1i} \prod_{j=1}^{n+1} (n+1)p_{2j} : \sum_{i=1}^{m+1} p_{1i} (V_{mi} - \theta_1) = 0; \right. \\ \left. \sum_{j=1}^{n+1} p_{2j} (W_{nj} - \theta_1 + \theta) = 0 \right\}.$$

**Remark 4.2.3.** For any given  $\theta$ , the IID-AEL ratio  $R^A(\theta_1, \theta)$  is always well defined because as  $n, m \rightarrow \infty$ , with probability tending to 1, the true value of  $\theta_1$  lies in the convex hull of  $V_{mi}$ 's and the true value of  $\theta_1 - \theta$  lies in the convex hull of  $W_{nj}$ 's.

Suppose  $\theta_1^0$  is the true value of  $\theta_1$ . In fact, for i.i.d.  $V_i$  and by construction of the AEL,  $\theta_1^0$  lies in the convex hull of  $V_i$ 's with probability tending to 1 as  $m$  approaches infinity.

This means that for  $\alpha_i \geq 0$ ,  $\sum_{i=1}^{m+1} \alpha_i = 1$ , we have  $\sum_{i=1}^{m+1} \alpha_i (V_i - \theta_1^0) = 0$ .

We can write

$$\begin{aligned} \sum_{i=1}^{m+1} \alpha_i (V_{mi} - \theta_1^0) &= \sum_{i=1}^{m+1} \alpha_i (V_{mi} - V_i) + \sum_{i=1}^{m+1} \alpha_i (V_i - \theta_1^0) \\ &= \sum_{i=1}^{m+1} \alpha_i (V_{mi} - V_i). \end{aligned}$$

For  $\beta_i$  such that  $\alpha_i = \frac{\beta_i}{\sqrt{m+1}}$ , as  $m \rightarrow \infty$ , we have

$$\begin{aligned} \sum_{i=1}^{m+1} \alpha_i (V_{mi} - \theta_1^0) &= \sum_{i=1}^{m+1} \frac{\beta_i}{\sqrt{m+1}} (V_{mi} - V_i) \\ &\leq \max_{1 \leq i \leq m+1} \beta_i \times \frac{1}{\sqrt{m+1}} \sum_{i=1}^{m+1} (V_{mi} - V_i) \\ &= o_p(1). \end{aligned}$$

because, as  $m \rightarrow \infty$ ,  $\frac{1}{\sqrt{m+1}} \sum_{i=1}^{m+1} (V_{mi} - V_i) = o_p(1)$  according to Theorem 4.2.1.

We can prove similar result for  $W_{nj}$ .

The IID-AEL log-ratio at  $(\theta_1, \theta)$  is then

$$\log R^A(\theta_1, \theta) = - \sum_{i=1}^{m+1} \log \{1 + \lambda_1^A (V_{mi} - \theta_1)\} - \sum_{j=1}^{n+1} \log \{1 + \lambda_2^A (W_{nj} - \theta_1 + \theta)\}, \quad (4.11)$$

where the Lagrange multipliers  $\lambda_1^A$  and  $\lambda_2^A$  are solutions to the ensuing equations

$$\frac{1}{m+1} \sum_{i=1}^{m+1} \frac{V_{mi} - \theta_1}{1 + \lambda_1^A (V_{mi} - \theta_1)} = 0, \quad (4.12)$$

and

$$\frac{1}{n+1} \sum_{j=1}^{n+1} \frac{W_{nj} - \theta_1 + \theta}{1 + \lambda_2^A (W_{nj} - \theta_1 + \theta)} = 0. \quad (4.13)$$

Profiling out  $\theta_1$ , we write  $\log r^A(\theta) \triangleq \log R^A(\hat{\theta}_1^A, \theta)$ , where  $\hat{\theta}_1^A = \max_{\theta_1} \log R^A(\theta_1, \theta)$ .

We can establish the following Wilks theorem for the IID-AEL ratio,  $r^A(\theta)$ .

**Theorem 4.2.3.** *Let  $\theta_0$  be the true value of  $\theta$ . Let  $t = m + n + 2$ . Assume the conditions of Theorem 4.2.1 hold and  $(m+1)/t \rightarrow \rho \in (0, 1)$  as  $t \rightarrow \infty$ . Then  $-2 \log r^A(\theta_0) \xrightarrow{D} \chi_1^2$ , as  $t \rightarrow \infty$ .*

Theorem 4.2.3 can be used to obtain, an asymptotic  $100(1 - \alpha)\%$  IID-AEL confidence interval for  $\theta$  as

$$I^A = \{\theta : -2 \log r^A(\theta) \leq \chi_1^2(\alpha)\}.$$

#### 4.2.3 I.i.d. mean empirical likelihood (IID-MEL)

In order to improve the poor accuracy of the EL methods for small sample sizes and multi-dimensional parameters, Liang et al. (2019) developed the mean empirical likelihood (MEL). The MEL ratio is based on pseudo-data, which consists of the pairwise means of the observed data. The MEL for two-sample comparison was developed in the context of i.i.d. random samples and its log-likelihood ratio converges in distribution to a weighted



sum of independent standard chi-square random variables with one degree of freedom. It can be applied to the samples  $V_m$  and  $W_n$  as they admit an i.i.d. representation. To obtain the IID-MEL, we apply the MEL to the samples  $V_m$  and  $W_n$  with  $V_{mi}$  and  $W_{nj}$  defined in equations (4.5) and (4.6). For this, we generate the pairwise data

$$\begin{cases} Z^V = \{(V_{mk} + V_{ml})/2; 1 \leq k \leq l \leq m\}, \\ Z^W = \{(W_{nk} + W_{nl})/2; 1 \leq k \leq l \leq n\}. \end{cases} \quad (4.14)$$

The pseudo samples obtained can be written as

$$\begin{cases} Z_1^V, Z_2^V, \dots, Z_M^V; M = m(m+1)/2, \\ Z_1^W, Z_2^W, \dots, Z_N^W; N = n(n+1)/2. \end{cases} \quad (4.15)$$

To define the two-sample IID-MEL, we let  $\mathbf{p}_1 = (p_{11}, p_{12}, \dots, p_{1M})'$  and  $\mathbf{p}_2 = (p_{21}, p_{22}, \dots, p_{2N})'$  be two probability vectors over the two samples  $Z_M^V$  and  $Z_N^W$ , respectively. The estimated IID-MEL ratio at  $(\theta_1, \theta)$  is

$$R^M(\theta_1, \theta) = \sup \left\{ \prod_{r=1}^M (M p_{1r}) \prod_{s=1}^N (N p_{2s}) : \sum_{r=1}^M p_{1r} Z_r^V = \theta_1; \sum_{s=1}^N p_{2s} Z_s^W = \theta_1 - \theta \right\}.$$

By the Lagrange multipliers method, we can derive the IID-MEL log-ratio at  $(\theta_1, \theta)$  as

$$\begin{aligned} \log R^M(\theta_1, \theta) = & - \sum_{r=1}^M \log \{1 + \lambda_1^M (Z_r^V - \theta_1)\} \\ & - \sum_{s=1}^N \log \{1 + \lambda_2^M (Z_s^W - \theta_1 + \theta)\}, \end{aligned} \quad (4.16)$$

where the Lagrange multipliers  $\lambda_1^M, \lambda_2^M$  are solutions to the following equations

$$\frac{1}{M} \sum_{r=1}^M \frac{Z_r^V - \theta_1}{1 + \lambda_1^M (Z_r^V - \theta_1)} = 0, \quad (4.17)$$

and

$$\frac{1}{N} \sum_{s=1}^N \frac{Z_s^W - \theta_1 + \theta}{1 + \lambda_2^{\mathcal{M}}(Z_s^W - \theta_1 + \theta)} = 0. \quad (4.18)$$

With  $\widehat{\theta}_1^{\mathcal{M}}$  being the value which maximizes  $\log R^{\mathcal{M}}(\theta_1, \theta)$  with respect to  $\theta_1$ , we can write  $\log r^{\mathcal{M}}(\theta) \triangleq \log R^{\mathcal{M}}(\widehat{\theta}_1^{\mathcal{M}}, \theta)$ . The following result for the IID-MEL ratio,  $r^{\mathcal{M}}(\theta_0)$  is proven.

**Theorem 4.2.4.** *Let  $\theta_0$  be the true value of  $\theta$ . Let  $s = m + n$ . Let  $V_i, W_j$  be defined by equations (4.3), (4.4). Assume the conditions of Theorem 4.2.1 hold and  $m/s = \delta \rightarrow \delta_0 \in (0, 1)$  as  $s \rightarrow \infty$ . Then  $-2 \log r^{\mathcal{M}}(\theta_0) / s \xrightarrow{\mathcal{D}} r \chi_1^2$ , as  $s \rightarrow \infty$ , where  $r = \left( \frac{\sigma_1^2}{\delta_0} + \frac{\sigma_2^2}{1-\delta_0} \right) / \left( \frac{\sigma_1^2}{\delta_0^2} + \frac{\sigma_2^2}{(1-\delta_0)^2} \right)$ ,  $\sigma_1^2 = \text{var}(V_i)$  and  $\sigma_2^2 = \text{var}(W_j)$ .*

Therefore, using Theorem 4.2.4, an asymptotic  $100(1-\alpha)\%$  IID-MEL confidence interval for  $\theta$  is given by

$$I^{\mathcal{M}} = \{ \theta : -2\widehat{r}^{-1} \log r^{\mathcal{M}}(\theta) / s \leq \chi_1^2(\alpha) \},$$

where  $\widehat{r}$  is the estimated value of  $r$  obtained by replacing  $\sigma_1^2$  and  $\sigma_2^2$  by their estimators  $\widehat{\sigma}_1^2$  and  $\widehat{\sigma}_2^2$ , respectively.

**Remark 4.2.4.** *For the variances  $\widehat{\sigma}_1^2$  and  $\widehat{\sigma}_2^2$ , the modified jackknife estimator (Stute, 1996) has been used in simulations, but any other consistent estimators can also be considered.*

### 4.3 Simulation Study

In this section, we report the results of a simulation study to compare the finite sample performance of the proposed IID-EL with the EL method in Wang and Wang (2001), denoted EL-WW, as well as the IID-AEL and the IID-MEL methods. Since the EL-WW performed better than the martingale based bootstrap (MBB) and the Efron's bootstrap (EB) methods (see Wang and Wang, 2001), we escape them in the simulation study for comparison.

Let us denote *ShiftedExp*( $\lambda, L$ ) as the shifted exponential distribution with shift parameter  $L$ , rate  $\lambda$  and the CDF  $G(x) = 1 - e^{-\lambda(x-L)}, x > L$ . Three simulated data have been considered.

- (i)  $X^0$  and  $Y^0$  are generated from the  $\mathcal{U}(0, 1)$  distribution and are censored by  $U$  and  $V$ , having the  $\mathcal{U}(0, c_0)$  distribution.
- (ii)  $X^0$  follows  $Exp(2)$ ,  $Y^0$ , the *ShiftedExp*(2, 1). The censoring variables  $U$  and  $V$  follow  $Exp(\lambda_1)$  and  $Exp(\lambda_2)$ , respectively.
- (iii)  $X^0$  is drawn from  $logNorm(0, 1)$ ,  $Y^0$  from  $\chi_3^2$ ,  $U$  from  $\mathcal{U}(c_1, 2c_1)$  and  $V$  from  $Exp(\lambda_3)$ .

The numbers  $c_0$ ,  $c_1$ ,  $\lambda_1$ ,  $\lambda_2$  and  $\lambda_3$  are used to control the desired censoring proportion. Based on the data set, the EL-WW-based, IID-EL-based, IID-AEL-based, and IID-MEL-based confidence intervals and their average lengths are calculated according to Theorem 2.1 (Wang and Wang, 2001), Theorem 4.2.2, Theorem 4.2.3, and Theorem 4.2.4 in Section 4.2 for 90% and 95% confidence levels. For each fixed value of  $c_0$ ,  $c_1$ ,  $\lambda_1$ ,  $\lambda_2$  and  $\lambda_3$  and sample sizes  $(m, n) = (10, 15)$ ,  $(15, 10)$ ,  $(25, 30)$ ,  $(30, 25)$  and  $(60, 60)$ , the process is repeated 10000 times. 10%, 25% and 40% censoring rates have been considered. The coverage probabilities and average lengths of confidence intervals are calculated and the simulation results are summarized in Tables 4.1-4.3 and Table 4.4-4.6 corresponding to 90% and 95% confidence levels, respectively.

Based on the tables we can make the following conclusions:

- 1) The coverage probabilities for all the methods tend to their nominal levels (0.90 and 0.95) as the sample sizes increase. Their performances are subject to the censoring proportions. As the censoring rate becomes higher, the coverage accuracy decreases. This behavior is uniform for the IID-EL, whereas it is violated sometimes with the EL-WW. For example, in Table 4.2, for  $(m, n) = (30, 25)$ , the coverage is better with 25% censoring rate than 10%. This fact shows the consistency of the IID-EL over the EL-WW. Also, the EL-WW attains the nominal levels with samples  $(m, n) = (25, 30)$ ,  $(m, n) = (30, 25)$  or  $(m, n) = (60, 60)$ , while the IID-EL reaches them quickly even with sample size  $(m, n) = (10, 15)$  or  $(m, n) = (15, 10)$ .
- 2) For both methods, the average lengths of the confidence intervals increase with the heavy censoring proportions, but decrease with the larger sample sizes. In most cases,

Table 4.1: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\theta$  at different censoring rates (CR) with 90% confidence level;  $X, Y \sim \mathcal{U}(0, 1)$  and  $U, V \sim \mathcal{U}(0, c_0)$

CR	$(m, n)$	EL-WW	IID-EL	IID-AEL	IID-MEL
0.10	(10, 15)	0.880 (0.349)	0.907 (0.384)	0.918 (0.459)	0.914 (0.402)
	(15, 10)	0.892 (0.340)	0.901 (0.381)	0.917 (0.461)	0.914 (0.402)
	(25, 30)	0.902 (0.253)	0.912 (0.261)	0.919 (0.295)	0.913 (0.265)
	(30, 25)	0.895 (0.224)	0.913 (0.258)	0.920 (0.294)	0.915 (0.262)
	(60, 60)	0.910 (0.167)	0.919 (0.178)	0.927 (0.192)	0.923 (0.179)
0.25	(10, 15)	0.861 (0.418)	0.896 (0.435)	0.917 (0.498)	0.911 (0.464)
	(15, 10)	0.844 (0.398)	0.887 (0.438)	0.915 (0.500)	0.909 (0.467)
	(25, 30)	0.856 (0.270)	0.899 (0.289)	0.918 (0.319)	0.910 (0.298)
	(30, 25)	0.908 (0.241)	0.910 (0.290)	0.916 (0.319)	0.914 (0.298)
	(60, 60)	0.907 (0.150)	0.915 (0.190)	0.920 (0.203)	0.917 (0.192)
0.40	(10, 15)	0.818 (0.452)	0.892 (0.534)	0.903 (0.577)	0.902 (0.584)
	(15, 10)	0.831 (0.417)	0.885 (0.530)	0.906 (0.571)	0.904 (0.572)
	(25, 30)	0.838 (0.340)	0.889 (0.361)	0.912 (0.381)	0.893 (0.385)
	(30, 25)	0.816 (0.281)	0.898 (0.366)	0.908 (0.385)	0.905 (0.389)
	(60, 60)	0.877 (0.212)	0.904 (0.232)	0.914 (0.242)	0.909 (0.240)

Table 4.2: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\theta$  at different censoring rates (CR) with 90% confidence level;  $X \sim Exp(2), Y \sim ShiftedExp(2, 1), U \sim Exp(\lambda_1)$  and  $V \sim Exp(\lambda_2)$

CR	$(m, n)$	EL-WW	IID-EL	IID-AEL	IID-MEL
0.10	(10, 15)	0.875 (0.414)	0.899 (0.546)	0.909 (0.628)	0.909 (0.580)
	(15, 10)	0.893 (0.355)	0.903 (0.514)	0.904 (0.577)	0.915 (0.570)
	(25, 30)	0.903 (0.317)	0.912 (0.475)	0.917 (0.509)	0.921 (0.497)
	(30, 25)	0.888 (0.329)	0.907 (0.469)	0.916 (0.521)	0.916 (0.510)
	(60, 60)	0.907 (0.274)	0.913 (0.331)	0.922 (0.376)	0.920 (0.343)
0.25	(10, 15)	0.866 (0.417)	0.882 (0.559)	0.905 (0.635)	0.892 (0.597)
	(15, 10)	0.862 (0.416)	0.866 (0.517)	0.902 (0.624)	0.889 (0.582)
	(25, 30)	0.860 (0.369)	0.903 (0.497)	0.910 (0.563)	0.905 (0.498)
	(30, 25)	0.901 (0.386)	0.907 (0.487)	0.912 (0.533)	0.915 (0.497)
	(60, 60)	0.904 (0.307)	0.903 (0.393)	0.915 (0.429)	0.908 (0.419)
0.40	(10, 15)	0.821 (0.440)	0.857 (0.575)	0.885 (0.651)	0.858 (0.623)
	(15, 10)	0.813 (0.457)	0.828 (0.590)	0.888 (0.694)	0.849 (0.601)
	(25, 30)	0.849 (0.366)	0.876 (0.499)	0.890 (0.523)	0.876 (0.555)
	(30, 25)	0.837 (0.385)	0.859 (0.489)	0.891 (0.512)	0.882 (0.520)
	(60, 60)	0.879 (0.355)	0.892 (0.411)	0.909 (0.436)	0.900 (0.455)

Table 4.3: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\theta$  at different censoring rates (CR) with 90% confidence level;  $X \sim \text{logNorm}(0, 1)$ ,  $Y \sim \chi_3^2$ ,  $U \sim \mathcal{U}(c_1, 2c_1)$  and  $V \sim \text{Exp}(\lambda_3)$

CR	$(m, n)$	EL-WW	IID-EL	IID-AEL	IID-MEL
0.10	(10, 15)	0.820 (1.104)	0.853 (1.266)	0.902 (1.694)	0.867 (1.469)
	(15, 10)	0.800 (0.859)	0.824 (1.031)	0.903 (1.523)	0.839 (1.195)
	(25, 30)	0.833 (0.666)	0.884 (0.733)	0.911 (0.909)	0.890 (0.853)
	(30, 25)	0.829 (0.490)	0.871 (0.637)	0.910 (0.819)	0.883 (0.729)
	(60, 60)	0.876 (0.204)	0.884 (0.225)	0.914 (0.289)	0.896 (0.275)
0.25	(10, 15)	0.803 (1.176)	0.836 (1.353)	0.887 (1.715)	0.853 (1.505)
	(15, 10)	0.814 (0.946)	0.839 (1.136)	0.868 (1.534)	0.839 (1.348)
	(25, 30)	0.815 (0.655)	0.864 (0.786)	0.900 (0.986)	0.869 (0.937)
	(30, 25)	0.795 (0.546)	0.835 (0.711)	0.899 (0.901)	0.859 (0.861)
	(60, 60)	0.857 (0.283)	0.865 (0.312)	0.902 (0.379)	0.869 (0.404)
0.40	(10, 15)	0.765 (1.227)	0.833 (1.375)	0.840 (1.744)	0.846 (1.567)
	(15, 10)	0.776 (0.995)	0.820 (1.195)	0.842 (1.671)	0.833 (1.546)
	(25, 30)	0.795 (0.792)	0.821 (0.872)	0.842 (1.114)	0.822 (1.029)
	(30, 25)	0.785 (0.598)	0.825 (0.748)	0.853 (0.970)	0.853 (0.999)
	(60, 60)	0.830 (0.289)	0.851 (0.318)	0.866 (0.433)	0.869 (0.444)

Table 4.4: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\theta$  at different censoring rates (CR) with 95% confidence level;  $X, Y \sim \mathcal{U}(0, 1)$  and  $U, V \sim \mathcal{U}(0, c_0)$

CR	$(m, n)$	EL-WW	IID-EL	IID-AEL	IID-MEL
0.10	(10, 15)	0.924 (0.432)	0.951 (0.450)	0.958 (0.551)	0.958 (0.487)
	(15, 10)	0.930 (0.445)	0.940 (0.459)	0.959 (0.553)	0.951 (0.488)
	(25, 30)	0.937 (0.298)	0.955 (0.310)	0.960 (0.353)	0.957 (0.318)
	(30, 25)	0.948 (0.245)	0.958 (0.309)	0.961 (0.353)	0.958 (0.317)
	(60, 60)	0.949 (0.185)	0.960 (0.212)	0.969 (0.229)	0.965 (0.214)
0.25	(10, 15)	0.920 (0.437)	0.947 (0.525)	0.957 (0.601)	0.953 (0.570)
	(15, 10)	0.922 (0.461)	0.938 (0.526)	0.949 (0.601)	0.950 (0.569)
	(25, 30)	0.927 (0.315)	0.947 (0.347)	0.954 (0.383)	0.952 (0.363)
	(30, 25)	0.934 (0.305)	0.952 (0.348)	0.956 (0.384)	0.954 (0.364)
	(60, 60)	0.948 (0.208)	0.957 (0.227)	0.963 (0.242)	0.960 (0.230)
0.40	(10, 15)	0.914 (0.499)	0.941 (0.649)	0.951 (0.698)	0.950 (0.719)
	(15, 10)	0.916 (0.555)	0.941 (0.639)	0.949 (0.691)	0.943 (0.701)
	(25, 30)	0.923 (0.365)	0.943 (0.438)	0.952 (0.461)	0.950 (0.480)
	(30, 25)	0.922 (0.396)	0.942 (0.444)	0.954 (0.467)	0.951 (0.485)
	(60, 60)	0.939 (0.257)	0.952 (0.281)	0.954 (0.292)	0.954 (0.297)

Table 4.5: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\theta$  at different censoring rates (CR) with 95% confidence level;  $X \sim Exp(2), Y \sim ShiftedExp(2, 1), U \sim Exp(\lambda_1)$  and  $V \sim Exp(\lambda_2)$

CR	$(m, n)$	EL-WW	IID-EL	IID-AEL	IID-MEL
0.10	(10, 15)	0.928 (0.499)	0.940 (0.589)	0.957 (0.630)	0.956 (0.608)
	(15, 10)	0.929 (0.538)	0.938 (0.568)	0.955 (0.645)	0.950 (0.609)
	(25, 30)	0.937 (0.403)	0.957 (0.481)	0.960 (0.586)	0.960 (0.522)
	(30, 25)	0.946 (0.392)	0.949 (0.478)	0.964 (0.588)	0.960 (0.540)
	(60, 60)	0.949 (0.301)	0.958 (0.361)	0.969 (0.417)	0.966 (0.401)
0.25	(10, 15)	0.912 (0.549)	0.939 (0.627)	0.953 (0.699)	0.940 (0.672)
	(15, 10)	0.909 (0.551)	0.918 (0.648)	0.947 (0.703)	0.942 (0.692)
	(25, 30)	0.923 (0.472)	0.950 (0.559)	0.954 (0.685)	0.954 (0.604)
	(30, 25)	0.925 (0.513)	0.934 (0.592)	0.959 (0.679)	0.951 (0.601)
	(60, 60)	0.943 (0.337)	0.957 (0.401)	0.960 (0.444)	0.958 (0.432)
0.40	(10, 15)	0.871 (0.576)	0.915 (0.664)	0.945 (0.719)	0.925 (0.736)
	(15, 10)	0.866 (0.583)	0.897 (0.653)	0.936 (0.726)	0.907 (0.715)
	(25, 30)	0.900 (0.508)	0.935 (0.691)	0.950 (0.709)	0.941 (0.716)
	(30, 25)	0.899 (0.533)	0.924 (0.688)	0.953 (0.702)	0.951 (0.711)
	(60, 60)	0.931 (0.405)	0.950 (0.500)	0.958 (0.508)	0.956 (0.558)



Table 4.6: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\theta$  at different censoring rates (CR) with 95% confidence level;  $X \sim \text{logNorm}(0, 1)$ ,  $Y \sim \chi_3^2$ ,  $U \sim \mathcal{U}(c_1, 2c_1)$  and  $V \sim \text{Exp}(\lambda_3)$

CR	$(m, n)$	EL-WW	IID-EL	IID-AEL	IID-MEL
0.10	(10, 15)	0.872 (1.607)	0.907 (1.849)	0.936 (2.305)	0.920 (2.118)
	(15, 10)	0.884 (1.417)	0.911 (1.588)	0.933 (2.114)	0.926 (1.860)
	(25, 30)	0.903 (0.904)	0.931 (1.176)	0.947 (1.359)	0.947 (1.383)
	(30, 25)	0.883 (0.877)	0.928 (1.053)	0.948 (1.253)	0.938 (1.188)
	(60, 60)	0.922 (0.459)	0.939 (0.524)	0.955 (0.605)	0.946 (0.621)
0.25	(10, 15)	0.850 (1.743)	0.902 (2.001)	0.933 (2.436)	0.929 (2.406)
	(15, 10)	0.871 (1.541)	0.905 (1.726)	0.930 (2.154)	0.925 (2.059)
	(25, 30)	0.878 (1.019)	0.897 (1.325)	0.933 (1.494)	0.932 (1.564)
	(30, 25)	0.880 (0.994)	0.901 (1.193)	0.935 (1.412)	0.920 (1.482)
	(60, 60)	0.901 (0.605)	0.918 (0.690)	0.937 (0.774)	0.934 (0.880)
0.40	(10, 15)	0.850 (1.759)	0.890 (2.041)	0.914 (2.467)	0.905 (2.410)
	(15, 10)	0.865 (1.726)	0.902 (1.934)	0.919 (2.363)	0.929 (2.404)
	(25, 30)	0.869 (1.156)	0.886 (1.503)	0.921 (1.738)	0.887 (1.806)
	(30, 25)	0.873 (1.473)	0.844 (1.377)	0.927 (1.565)	0.889 (1.767)
	(60, 60)	0.874 (0.728)	0.887 (0.838)	0.929 (0.935)	0.917 (1.121)

IID-EL based confidence interval performs better than these of the EL-WW-based confidence intervals. They provide better coverage probability, but the interval lengths are slightly wider.

- 3) The IID-AEL and the IID-MEL boosted the coverage considerably for small samples and performed almost equally. For both methods, the average length is bigger than this of IID-EL, resulting in higher coverages. Their usefulness is emphasized when the censoring rate increases as the coverage probabilities get closer to the fixed nominal levels.
- 4) When  $(m, n) = (60, 60)$ , and in some other cases, The proposed EL confidence intervals are over-covered, but this behavior vanishes with heavy censored data.

Based on the simulations results, we advise to use the proposed IID-EL method for constructing confidence intervals for the difference of two means under right censoring as its coverage probabilities are the closest to the confidence levels. We can use either IID-AEL or IID-MEL when the samples are small, and the censoring proportion is significantly high.

#### 4.4 Real Application

In this section, we apply the proposed method to the famous Primary Biliary Cirrhosis (PBC) data set. The data set can be found in Appendix D of Fleming and Harrington (1991). This data is from the Mayo Clinic trial in PBC of the liver, conducted between 1974 and 1984. From the 424 patients eligible for the trial, 312 consent to take part in a double-blinded randomized trial, and we consider these 312 patients for this study. They were divided into two groups receiving the drug D-penicillamine (DPCA) or a placebo. As of July 1986, disease and survival status, censoring status and a lot of covariates measurements were recorded, 125 patients had died (11 deaths not due to PBC), eight had been lost to follow-up, and 19 had undergone a liver transplant. These latter are considered as censored for this study. For each group, we consider the subgroup consisting of the male patients. Thus, the DPCA group has 21 patients with a censoring rate of 33%, and the placebo group

15 patients and 36% censoring. We are interested in the difference of the mean survival times  $\theta$ , between the two groups. The nonparametric maximum likelihood estimator (NPMLE) for  $\theta$  is  $\hat{\theta} = 860$  days and the 90% and 95% confidence intervals using the EL-WW, IID-EL, IID-AEL, and IID-MEL methods are summarized in Table 4.7. Although the IID-EL, IID-AEL, and IID-MEL intervals are wider than the EL-WW one, all contain zero. We conclude that there is no statistically significant difference between the mean survival times in the two groups. This result confirms the conclusion of previous studies, i.e, there are no detectable difference between the distributions of survival times for the DPCA and placebo groups (see Fleming and Harrington, 1991).

Table 4.7: 90% and 95% Confidence intervals for  $\theta$  for the PBC data

	$1 - \alpha = 0.90$	$1 - \alpha = 0.95$
EL-WW	(-165.38, 1502.31)	(-365.41, 1567.23)
IID-EL	(-181.67, 1526.01)	(-386.22, 1591.96)
IID-AEL	(-255.53, 1578.67)	(-452.08, 1623.69)
IID-MEL	(-246.87, 1607.29)	(-419.20, 1709.26)

## 4.5 Conclusions

In this chapter, we propose inference on the mean difference for right-censored data using a combination of EL and i.i.d. representation technique (IID-EL). We prove that the empirical log-likelihood ratio statistic converges to a chi-squared distribution asymptotically, i.e., Wilks' theorem is preserved contrary to the plug-in EL method. By inverting the EL ratio statistic, we construct a confidence interval for the mean difference. The proposed method (IID-EL) and its calibrations (IID-AEL, IID-MEL) have some advantages over the plug-in EL, including better coverage probability. The IID-EL has practical value, as shown by a real data application.

## CHAPTER 5

### WEIGHTED EMPIRICAL LIKELIHOOD INFERENCE FOR THE DIFFERENCE BETWEEN THE AREAS UNDER TWO CORRELATED ROC CURVES WITH RIGHT-CENSORED DATA

#### 5.1 Background

The area under the ROC curve (AUC) is a synthetic index calculated for ROC curves. The receiver operating characteristic (ROC) curve is developed initially during World War II to analyze radar receivers. Its primary purpose was to differentiate enemy aircraft from a signal noise. It has been quickly adapted to several areas such as psychology, medicine, epidemiology, radiology, etc. The ROC curve can be used for any analysis method that aims to distinguish two populations: one with a condition and the other one without it. That is why it is prolific in a laboratory or diagnostic tests, screening tests that try to discriminate disease and non-disease patients, and in analyzing the accuracy of statistical models that classify subjects into one or two groups (linear regression, linear discriminant analysis). An excellent summary of recent studies and different applications of the ROC curve is provided by Pepe (2003). We define here the ROC curve and the AUC in the framework of diagnostic medicine.

Let  $F$  and  $G$  be the cumulative distribution functions (CDF) of non-disease and disease populations, respectively. Let  $X$  and  $Y$  be the response variables of a continuous-scale diagnostic test for a non-disease and a disease patient, respectively. Patients with a response greater than a given threshold  $c$  are considered as ‘disease’ and those with a response smaller than  $c$  are ‘non-disease’. The true positive rate (TPR) or sensitivity is the probability that the test detects the disease when it is present, and the false positive rate (FPR), the complement of specificity, is the probability that the test detects the disease when it is not present. Sensitivity and specificity of the test are defined as  $Se(c) = P(Y > c) = 1 - G(c)$

and  $Sp(c) = P(X \leq c) = F(c)$ . The ROC curve is the plot of  $Se(c)$  against  $1 - Sp(c)$  for  $-\infty \leq c \leq \infty$ , or equivalently as a plot of  $ROC(p) = 1 - G(F^{-1}(1 - p))$ , for  $p \in [0, 1]$ , where  $F^{-1}(p) = \inf \{x \in \mathbb{R} : F(x) \geq p\}$ . The area under the ROC curve (denoted  $\Delta$ ) is then defined as

$$\Delta = \int_0^1 ROC(p) dp. \quad (5.1)$$

Bamber (1975) proved that  $\Delta = P(X \leq Y)$ . Thereby, the AUC can be interpreted as the probability that, among two subjects chosen at random, a disease patient and a non-disease patient, the value of the diagnostic test marker is higher for the disease patient than for the non-disease patient. Therefore, an AUC of 0.5 indicates that the marker is non-informative. An increase in AUC indicates an improvement in discriminatory abilities, with a maximum of 1. It provides options to compare two ROC curves generated from independent groups or paired subjects.

Pepe and Cai (2004) defined the ROC curve as the probability distribution of placement values. For a given  $Y$  from the disease population, the placement value is defined as  $\mathcal{U} = 1 - F(Y)$ .  $\mathcal{U}$  represents the proportion of non-diseased subjects with their test values larger than  $Y$ , and so marks the placement of  $Y$  within the non-diseased population. Based on this relationship, we can write

$$\Delta = E(1 - \mathcal{U}) = E(F(Y)). \quad (5.2)$$

It often happens that two or more diagnostic tests are compared to judge their discriminating capacity in the face of the patient's condition. It is also possible that two doctors will perform a patient's assessment. The data's correlated nature must be taken into account in the statistical analysis of differences between areas when two or more empirical curves are constructed from tests based on the same individuals. The design using the same individuals is, in general, more precise because it controls the inter-patient variation.

For complete data, many authors have considered parametric, semi-parametric, and nonparametric methods for comparing two AUCs. The parametric approach has been proposed alternately by Dorfman and Alf Jr. (1969), McClish (1989), and later Metz et al.

(1998). Their method assumes that the samples have a binormal distribution. The area under an empirical ROC curve is equal to the Mann-Whitney two-sample statistic. Delong et al. (1988), noting that the Mann-Whitney statistic is a generalized  $U$ -statistic, proposed a test statistic having a standard normal distribution that can be used to construct CI for the difference  $\Delta$ . All the above mentioned methods are associated with a massive computation. Another nonparametric method that has proven itself in reducing the computation burden is the EL method. To avoid estimating link variables associated with the EL method, Yang and Zhao (2013) developed the jackknife empirical likelihood (JEL) method for the difference of two correlated continuous-scale ROC curves. Zhang and Zhang (2014) developed a semiparametric EL and EL CIs for the difference between two correlated AUCs. To assess the discriminating power of a diagnostic test with three ordinal groups, An and Zhao (2017) proposed the JEL for the difference of two volumes under correlated ROC surfaces.

While all these methods are developed for uncensored data, the literature does not show a single published article comparing two correlated AUCs for the censored case. Chraznowski (2014) proposed the weighted EL (WEL) for a single AUC with right-censored data and established its asymptotic properties. Our work is the extension of Chraznowski's one sample result in two samples.

In the present chapter, we develop the two-sample WEL method for the difference of two areas under two correlated ROC curves using the placement value approach. Moreover, we apply an adjustment to boost the coverage rate of confidence intervals for small samples.

The chapter is organized as follows. In Section 5.2, we introduce the notations and establish the asymptotic property of the proposed NA, WEL methods and the adjustment denoted AWEL. Simulation studies are conducted in Section 5.3. We illustrate the proposed method by a real example in Section 5.4, and a brief conclusion is given in Section 5.5. The proofs are provided in the Appendix D.

## 5.2 Main Results

We use similar notations as Wang et al. (2009) and Chrzanowski (2014) did. Let  $(T_1, T_2)$  be a pair of diagnostic tests with continuous outcomes. Both tests are performed on  $m$  non-disease subjects and  $n$  disease subjects. Let  $(X_1, X_2)$  and  $(Y_1, Y_2)$  be the population of non-disease and disease subjects, respectively. Let  $(X_1^0, X_2^0)$  and  $(Y_1^0, Y_2^0)$  be the test results for the non-diseased and diseased subjects, respectively.  $\{(X_{11}^0, X_{21}^0), (X_{12}^0, X_{22}^0), \dots, (X_{1m}^0, X_{2m}^0)\}$  and  $\{(Y_{11}^0, Y_{21}^0), (Y_{12}^0, Y_{22}^0), \dots, (Y_{1n}^0, Y_{2n}^0)\}$  represent positive bivariate random samples from  $(X_1^0, X_2^0)$  and  $(Y_1^0, Y_2^0)$  with the cumulative distribution functions (CDF)  $F(x_1, x_2)$  and  $G(y_1, y_2)$ , respectively. These two samples are randomly right-censored by the sets of random variables  $\{U_{11}, U_{12}, \dots, U_{1m}\}$ ,  $\{U_{21}, U_{22}, \dots, U_{2m}\}$ ,  $\{V_{11}, V_{12}, \dots, V_{1n}\}$ , and  $\{V_{21}, V_{22}, \dots, V_{2n}\}$  with CDF  $K_1$ ,  $K_2$ ,  $Q_1$ , and  $Q_2$ , respectively. Due to this censoring scheme, we cannot observe directly  $(X_{1i}^0, X_{2i}^0)$  and  $(Y_{1j}^0, Y_{2j}^0)$  but, instead we observe  $(X_{1i}, X_{2i}, \delta_{1i}, \delta_{2i})$  and  $(Y_{1j}, Y_{2j}, \eta_{1j}, \eta_{2j})$ ,

where

$$X_{1i} = \min(X_{1i}^0, U_{1i}), \quad \delta_{1i} = I(X_{1i}^0 \leq U_{1i}), \quad i = 1, 2, \dots, m,$$

$$X_{2i} = \min(X_{2i}^0, U_{2i}), \quad \delta_{2i} = I(X_{2i}^0 \leq U_{2i}), \quad i = 1, 2, \dots, m,$$

$$Y_{1j} = \min(Y_{1j}^0, V_{1j}), \quad \delta_{1j} = I(Y_{1j}^0 \leq V_{1j}), \quad j = 1, 2, \dots, n,$$

$$Y_{2j} = \min(Y_{2j}^0, V_{2j}), \quad \delta_{2j} = I(Y_{2j}^0 \leq V_{2j}), \quad j = 1, 2, \dots, n.$$

All the variables  $X_{ki}^0$ ,  $U_{ki}$ ,  $Y_{kj}^0$ , and  $V_{kj}$  are mutually independent, for  $i = 1, 2, \dots, m$ ,  $j = 1, 2, \dots, n$ ,  $k = 1, 2$ , and  $I(\cdot)$  is the indicator function.

Let  $F_1(x_1) = F(x_1, \infty)$ ,  $F_2(x_2) = F(\infty, x_2)$ ,  $G_1(y_1) = G(y_1, \infty)$ , and  $G_2(y_2) = G(\infty, y_2)$  be the marginal distributions for  $X_1^0, X_2^0, Y_1^0$ , and  $Y_2^0$ , respectively.  $F_k$ ,  $G_k$ ,  $K_k$ , and  $Q_k$ , for  $k = 1, 2$  are unknown. Let  $\tau_f = \inf\{t : f(t) = 1\}$ . We assume  $\tau_{F_k} \leq \tau_{K_k}, \tau_{G_k} \leq \tau_{Q_k}$  and without loss of generality  $\tau_{F_k} \leq \tau_{G_k}$ ,  $k = 1, 2$ . Based on the placement value and denoting

$\Delta_k$  the AUC corresponding to the test  $T_k$ , we have

$$\Delta_k = E_{G_k} \{F_k(Y_{k1})\}. \quad (5.3)$$

Setting  $Z_{kj} = F_k(Y_{kj})$ , we obtain a simpler form  $\Delta_k = E_{G_k} \{Z_{k1}\}$ . We are interested in the difference  $\Delta = \Delta_1 - \Delta_2$ . For inference on  $\Delta$ , all the unknown distributions  $F_k$ ,  $G_k$ ,  $K_k$ , and  $Q_k$  will be replaced by their Kaplan-Meier estimators

$$\begin{aligned} 1 - \widehat{F}_k(t) &= \prod_{i=1}^m \left[ \frac{m-i}{m-i+1} \right]^{I(X_{(ki)} \leq t, \delta_{(ki)}=1)}, \\ 1 - \widehat{G}_k(t) &= \prod_{j=1}^n \left[ \frac{n-j}{n-j+1} \right]^{I(Y_{(kj)} \leq t, \eta_{(kj)}=1)}, \\ 1 - \widehat{K}_k(t) &= \prod_{i=1}^m \left[ \frac{m-i}{m-i+1} \right]^{I(X_{(ki)} \leq t, \delta_{(ki)}=0)}, \\ 1 - \widehat{Q}_k(t) &= \prod_{j=1}^n \left[ \frac{n-j}{n-j+1} \right]^{I(Y_{(kj)} \leq t, \eta_{(kj)}=0)}, \end{aligned}$$

where  $\{X_{(k1)}, X_{(k2)}, \dots, X_{(km)}\}$  (respectively  $\{Y_{(k1)}, Y_{(k2)}, \dots, Y_{(kn)}\}$ ) are the ordered values of the sample  $X_{ki}$  (respectively the sample  $Y_{kj}$ ) and  $\{\delta_{(k1)}, \delta_{(k2)}, \dots, \delta_{(km)}\}$  (respectively  $\{\eta_{(k1)}, \eta_{(k2)}, \dots, \eta_{(kn)}\}$ ) the corresponding values of  $\delta_{ki}$  associated to  $X_{ki}$  (respectively  $\eta_{kj}$  associated to  $Y_{kj}$ ). Note that we set  $\widehat{F}_k(X_{(km)}) = \widehat{G}_k(Y_{(kn)}) = \widehat{K}_k(X_{(km)}) = \widehat{Q}_k(Y_{(kn)}) = 1$  to make  $\widehat{F}_k$ ,  $\widehat{G}_k$ ,  $\widehat{K}_k$ , and  $\widehat{Q}_k$  proper distributions, in case they are not. To estimate the AUC we use the estimator developed by Wang et al (2009) and define

$$\widehat{\Delta}_k = \int_0^{X_{(km)}} \widehat{F}_k(t) d\widehat{G}_k(t) + 1 - \widehat{G}_k(X_{(km)}).$$

Let  $H_k(t) = P(X_{k1} \leq t)$ ,  $L_k(t) = P(Y_{k1} \leq t)$ ,  $\Lambda_{F_k}(t) = \int_0^t dF_k(s)/(1 - F_k(s-))$ ,  $\Lambda_{G_k}(t) = \int_0^t dG_k(s)/(1 - G_k(s-))$ . The following theorem describes the asymptotic property of the estimator  $\widehat{\Delta}_k$  and can be extended to obtain this of  $\widehat{\Delta}$ .



**Theorem 5.2.1.** (Wang et al., 2009, Theorem 2.2). Let  $\Delta_k^0$  be the true value of  $\Delta_k$ . Assume that the regularity conditions in the Appendix hold. Then

$$\sqrt{m+n} \left( \widehat{\Delta}_k - \Delta_k^0 \right) \xrightarrow{\mathcal{D}} \mathcal{N}(0, \sigma_k^2),$$

where  $\sigma_k^2 = (1+1/\rho)\sigma_{xk}^2 + (1+\rho)\sigma_{yk}^2$ ,  $\rho = \lim(m/n)$ ,

$$\begin{aligned} \sigma_{xk}^2 &= \int_0^{\tau_{F_k}} \left\{ \int_t^{\tau_{F_k}} (1 - F_k(s)) dG_k(s) \right\}^2 \frac{1 - F_k(t-)}{1 - F_k(t)} \frac{1}{1 - H_k(t)} d\Lambda_{F_k}(t), \\ \sigma_{yk}^2 &= \int_0^{\tau_{F_k}} \left\{ F_k(t)(1 - G_k(t)) - \int_t^{\tau_{F_k}} F_k(s) dG_k(s) - (1 - G_k(\tau_{F_k})) \right\}^2 \\ &\quad \times \frac{1 - G_k(t-)}{1 - G_k(t)} \frac{1}{1 - L_k(t)} d\Lambda_{G_k}(t). \end{aligned} \quad (5.4)$$

A consistent estimator of  $\sigma_k^2$  can be obtained by replacing  $F_k$  and  $G_k$  by their Kaplan-Meier estimators,  $H_k$  and  $L_k$  by their empirical counterparts  $H_{km} = (1/m) \sum_{i=1}^m I(X_{ki} \leq t)$  and  $L_{kn} = (1/n) \sum_{j=1}^n I(Y_{kj} \leq t)$ , and the cumulative hazard functions  $\Lambda_{F_k}$  and  $\Lambda_{G_k}$  by their Nelson-Aalen estimators  $\widehat{\Lambda}_{F_k}$  and  $\widehat{\Lambda}_{G_k}$ .

### 5.2.1 Normal approximation (NA)

For the large sample, having a consistent estimate  $\widehat{\Delta}$  for  $\Delta$  and using the Central Limit Theorem we can establish NA asymptotic results for  $\Delta$ . Let

$$J_{mx_k}(t) = I(0 \leq t \leq X_{(km)}), J_{ny_k} = I(0 \leq t \leq Y_{(kn)}),$$

$$H_{km}^1 = (1/m) \sum_{i=1}^m I(X_{ki} \leq t, \delta_{(ki)} = 1), L_{kn}^1 = (1/n) \sum_{j=1}^n I(Y_{kj} \leq t, \eta_{(kj)}),$$

$$M^{F_k}(t) = \sqrt{m} \left( H_{km}^1 - \int_0^t (1 - H_{km}(s-)) d\Lambda^{F_k}(s) \right),$$

$$M^{G_k}(t) = \sqrt{n} \left( L_{kn}^1 - \int_0^t (1 - L_{kn}(s-)) d\Lambda^{G_k}(s) \right),$$

$$\alpha_{1k} = \int_0^{X_{(km)}} \left\{ \int_t^{X_{(km)}} (1 - F_k(s)) dG_k(s) \right\} \frac{1 - \widehat{F}_k(t-)}{1 - F_k(t)} \frac{J_{mx_k}(t)}{1 - H_{km}(t-)} dM^{F_k}(t), \quad (5.5)$$

$$\beta_{1k} = \int_0^{X_{(km)}} \left\{ F_k(t)(1 - G_k(t)) - \int_t^{X_{(km)}} F_k(s) dG_k(s) - (1 - G_k(X_{(km)})) \right\} \times \frac{1 - \widehat{G}_k(t-)}{1 - G_k(t)} \frac{J_{ny_k}}{1 - L_{kn}(t-)} dM^{G_k}(t). \quad (5.6)$$

Using Wang et al. (2009) estimator, we can derive the asymptotic distribution of the difference  $\widehat{\Delta} = \widehat{\Delta}_1 - \widehat{\Delta}_2$  according to the following theorem.

**Theorem 5.2.2.** *Let  $\Delta^0$  be the true value of  $\Delta$ . Assume that the regularity conditions in the Appendix hold. Then*

$$\begin{aligned} \sqrt{m+n} \left( \widehat{\Delta} - \Delta^0 \right) &\xrightarrow{\mathcal{D}} \mathcal{N}(0, \sigma^2), \\ \sigma^2 &= \sigma_1^2 + \sigma_2^2 - 2\sigma_{12}^2, \\ \sigma_{12}^2 &= (1 + 1/\rho)\text{Cov}(\alpha_{11}, \alpha_{12}) + (1 + \rho)\text{Cov}(\beta_{11}, \beta_{12}), \rho = \lim(m/n), \end{aligned}$$

where  $\sigma_k^2$  is defined in Theorem 5.2.1.

Denoting  $\widehat{\sigma}^2$  a consistent estimator for  $\sigma^2$  and using Theorem 5.2.2, a  $100(1 - \alpha)\%$  normal approximation-based confidence interval for  $\Delta$  can be constructed as follows.

$$I_{NA} = \left( \widehat{\Delta} - z_{1-\alpha/2} \sqrt{\frac{\widehat{\sigma}^2}{m+n}}, \widehat{\Delta} + z_{1-\alpha/2} \sqrt{\frac{\widehat{\sigma}^2}{m+n}} \right),$$

where  $z_{1-\alpha/2}$  is the  $(1 - \alpha/2)$ th quantile of the standard normal distribution.

## 5.2.2 Weighted empirical likelihood (WEL)

The computation of the normal based CI requires a great amount of calculation due to the complicated form of the variance that need to be estimated. We turn to the WEL that does not need a variance estimation as any EL method. We cannot define the WEL on  $Z_{kj}$ 's since they are unknown. They are replaced by  $\widehat{Z}_{kj} = \widehat{F}_k(Y_{kj})$ . We defined here the estimated WEL (EWEL) for the true value  $\Delta^0$  of  $\Delta$ , in the sense of Ren (2001, 2008). Let us consider

the weights  $\omega_{kj} = n\Delta\widehat{G}_k(Y_{kj})$  based on the Kaplan-Meier jumps of the estimator  $\widehat{G}_k$ . They represent the importance of each observation  $\widehat{Z}_{kj}$  in the pseudo sample  $\widehat{Z}_{k1}, \widehat{Z}_{k2}, \dots, \widehat{Z}_{kn}$ , for  $k = 1, 2$ . Moreover, Wang et al. (2009) estimator is a weighted mean of those pseudo-values:  $\widehat{\Delta}_k = \frac{1}{\sum_{l=1}^n \omega_{kl}} \sum_{j=1}^n \omega_{kj} \widehat{Z}_{kj}$ . Although the sum of the weights is  $n$ , for the rest of the chapter and to avoid any ambiguity we will denote the sample size by  $n$  and the sum of the weights  $\sum_{l=1}^n \omega_{kl}$  by  $\sum \omega_{kl}$ .

Let  $(p_{k1}, p_{k2}, \dots, p_{kn})'$  be two probability vectors,  $k = 1, 2$ . We have

$$EWEL(\Delta^0) = \sup \left\{ \prod_{k=1}^2 \prod_{j=1}^n p_{kj}^{\omega_{kj}} : \sum_{j=1}^n \omega_{kj} p_{kj} = 1, \sum_{j=1}^n \omega_{kj} p_{kj} (\widehat{Z}_{kj} - \Delta_k^0) = 0; \right. \\ \left. \sum_{j=1}^n \omega_{1j} p_{1j} \widehat{Z}_{1j} - \sum_{j=1}^n \omega_{2j} p_{2j} \widehat{Z}_{2j} = \Delta^0 \right\}.$$

By the technique of Lagrange multipliers, we can derive that the estimated weighted empirical log-likelihood ratio (EWELLR) for  $\Delta^0$ , denoted  $R(\Delta^0)$  verifies

$$l(\Delta^0) = -2 \log R(\Delta^0) \\ = 2 \left( \sum_{j=1}^n \omega_{1j} \log\{1 + 2\lambda(\widehat{Z}_{1j} - \Delta_1^0)\} + \sum_{j=1}^n \omega_{2j} \log\{1 - 2\lambda(\widehat{Z}_{2j} - \Delta_2^0)\} \right),$$

where  $\lambda = \lambda(\Delta^0)$ ,  $\Delta_1^0$ , and  $\Delta_2^0$  satisfy the following system of equations

$$\begin{cases} \frac{1}{\sum w_{1l}} \sum_{j=1}^n \frac{\omega_{1j}(\widehat{Z}_{1j} - \Delta_1^0)}{1 + 2\lambda(\widehat{Z}_{1j} - \Delta_1^0)} = 0, \\ \frac{1}{\sum w_{2l}} \sum_{j=1}^n \frac{\omega_{2j}(\widehat{Z}_{2j} - \Delta_2^0)}{1 - 2\lambda(\widehat{Z}_{2j} - \Delta_2^0)} = 0, \\ \frac{1}{\sum w_{1l}} \sum_{j=1}^n \frac{\omega_{1j} \widehat{Z}_{1j}}{1 + 2\lambda(\widehat{Z}_{1j} - \Delta_1^0)} - \frac{1}{\sum w_{2l}} \sum_{j=1}^n \frac{\omega_{2j} \widehat{Z}_{2j}}{1 - 2\lambda(\widehat{Z}_{2j} - \Delta_2^0)} = \Delta^0. \end{cases} \quad (5.7)$$

Due to the dependent nature of  $\widehat{Z}_{k1}, \widehat{Z}_{k2}, \dots, \widehat{Z}_{kn}$ , the asymptotic distribution of the EWELLR statistic is a scaled- $\chi_1^2$ , as stated in the following theorem.

**Theorem 5.2.3.** *Recall that  $\Delta^0$  is the true value of  $\Delta$ . Let  $r(\Delta^0) = (m+n)(S_1^2 + S_2^2)/(n\sigma^2)$ .*

Assume that the regularity conditions in the Appendix hold. Then

$$r(\Delta^0)l(\Delta^0) \xrightarrow{\mathcal{D}} \chi_1^2, \quad \text{as } n \rightarrow \infty,$$

where  $S_k^2 = \frac{1}{\sum \omega_{kl}} \sum_{j=1}^n \omega_{kj} (\widehat{Z}_{kj} - \Delta_k^o)^2$ ,  $\sigma^2$  is defined in Theorem 5.2.2, and  $\chi_1^2$  is a standard chi-squared random variable with one degree of freedom.

Thus, using Theorem 5.2.3, an asymptotic  $100(1 - \alpha)\%$  EL confidence interval for  $\Delta$  is given by

$$I_{WEL} = \{\Delta : \widehat{r}(\Delta)l(\Delta) \leq \chi_1^2(\alpha)\},$$

where  $\chi_1^2(\alpha)$  is the upper  $\alpha$ -quantile of the distribution of  $\chi_1^2$ . The estimate  $\widehat{r}(\Delta)$  is obtained by replacing  $\sigma_1^2, \sigma_2^2$  and  $\sigma^2$  by consistent estimates  $\widehat{\sigma}_1^2, \widehat{\sigma}_2^2$  and  $\widehat{\sigma}^2$  in  $r(\Delta)$ .

### 5.2.3 Adjusted weighted empirical likelihood (AWEL)

The adjusted empirical likelihood (AEL) introduced by Chen et al. (2008) is a calibration method, whose primary purpose is to make sure that the EL estimation equations always have a solution. It is beneficial for small samples as it solves the ‘empty set’ problem. This is done by adding a pseudo-observation to the data, and by doing so, widen the confidence interval and provide better coverage probability of confidence intervals. Following Chen et al. (2008)’s suggestion, we define the  $(n + 1)$ th observation  $\widehat{Z}_{kn+1}$  as

$$\widehat{Z}_{kn+1} = -\frac{a_n}{n} \sum_{j=1}^n \widehat{Z}_{kj}, \quad k = 1, 2,$$

where  $a_n = \max(1, \log(n)/2)$ . Let  $\omega_{kj} = (n+1)\Delta\widehat{G}_k(Y_{kj})$ . Based on the  $(n+1)$  observations, we define the estimated adjusted WEL (EAWEL) likelihood ratio as

$$EAWEL(\Delta^0) = \sup \left\{ \prod_{k=1}^2 \prod_{j=1}^{n+1} p_{kj}^{\omega_{kj}} : \sum_{j=1}^{n+1} \omega_{kj} p_{kj} = 1, \sum_{j=1}^{n+1} \omega_{kj} p_{kj} (\widehat{Z}_{kj} - \Delta_k^0) = 0; \right. \\ \left. \sum_{j=1}^{n+1} \omega_{1j} p_{1j} \widehat{Z}_{1j} - \sum_{j=1}^{n+1} \omega_{2j} p_{2j} \widehat{Z}_{2j} = \Delta^0 \right\}.$$

As in the previous section, with the method of Lagrange multipliers, we can show that estimated adjusted weighted empirical log-likelihood ratio (EAWELLR) for  $\Delta^0$ , denoted  $R^A(\Delta^0)$  verifies

$$l^A(\Delta^0) = -2 \log R^A(\Delta^0) \\ = 2 \left( \sum_{j=1}^{n+1} \omega_{1j} \log\{1 + 2\lambda^A(\widehat{Z}_{1j} - \Delta_1^0)\} + \sum_{j=1}^{n+1} \omega_{2j} \log\{1 - 2\lambda^A(\widehat{Z}_{2j} - \Delta_2^0)\} \right),$$

where  $\lambda^A = \lambda^A(\Delta^0)$ , the Lagrange multiplier,  $\Delta_1^0$ , and  $\Delta_2^0$  are solutions of the following equations

$$\begin{cases} \frac{1}{\sum w_{1l}} \sum_{j=1}^{n+1} \frac{\omega_{1j}(\widehat{Z}_{1j} - \Delta_1^0)}{1 + 2\lambda^A(\widehat{Z}_{1j} - \Delta_1^0)} = 0, \\ \frac{1}{\sum w_{2l}} \sum_{j=1}^{n+1} \frac{\omega_{2j}(\widehat{Z}_{2j} - \Delta_2^0)}{1 - 2\lambda^A(\widehat{Z}_{2j} - \Delta_2^0)} = 0, \\ \frac{1}{\sum w_{1l}} \sum_{j=1}^{n+1} \frac{\omega_{1j} \widehat{Z}_{1j}}{1 + 2\lambda^A(\widehat{Z}_{1j} - \Delta_1^0)} - \frac{1}{\sum w_{2l}} \sum_{j=1}^{n+1} \frac{\omega_{2j} \widehat{Z}_{2j}}{1 - 2\lambda^A(\widehat{Z}_{2j} - \Delta_2^0)} = \Delta^0. \end{cases}$$

The first order asymptotic properties of the EL are preserved by the AEL. We establish the following theorem for the AWEL.

**Theorem 5.2.4.** *Assume that the regularity conditions in the Appendix hold. Then*

$$r(\Delta^0)l^A(\Delta^0) \xrightarrow{D} \chi_1^2, \quad \text{as } n \rightarrow \infty.$$

Therefore, an asymptotic  $100(1 - \alpha)\%$  AWEL confidence interval for  $\Delta$  is constructed as follows

$$I^A = \{\Delta : \widehat{r}(\Delta)l^A(\Delta) \leq \chi_1^2(\alpha)\}.$$

### 5.3 Simulation Study

Monte Carlo simulations have been conducted to assess the performance of the proposed methods for finite and moderate samples. For all the following simulations, five sample sizes  $(m, n) = (50, 50), (70, 50), (70, 70), (100, 70), (100, 100)$  have been considered. We generate 5000 pairs of samples  $X^0$  of size  $m$ , and 5000 pairs of samples  $Y^0$  of size  $n$  from the bivariate exponential with mean  $(1, 1)$  and bivariate exponential with mean  $(c_{G_1}, c_{G_2})$ . The correlation between paired test outcomes is set as  $r = 0$ ,  $r = 0.3$ , and  $r = 0.7$ . The censoring variables  $U_1, U_2, V_1$  and  $V_2$  are generated from *Exponential*( $c_{K_1}$ ), *Exponential*( $c_{K_2}$ ), *Exponential*( $c_{Q_1}$ ), and *Exponential*( $c_{Q_2}$ ), respectively. In each case, the parameters  $c_{G_1}, c_{G_2}, c_{K_1}, c_{K_2}, c_{Q_1}$  and  $c_{Q_2}$  are suitably chosen to accommodate the different correlations and censoring rates. A combination of three censoring rates (CR), 10%, 25%, and 40% have been used. CR1=(0.10, 0.10, 0.10, 0.10), CR2=(0.10, 0.25, 0.10, 0.25), CR3=(0.40, 0.25, 0.40, 0.25). The components of CR $k$ ,  $k = 1, 2, 3$ , correspond to the censoring rates for the variables  $U_1, U_2, V_1$  and  $V_2$  in this order. For all the generated samples, we considered the 95% CIs and the average lengths of CIs for  $\Delta = -0.1, 0.05$  and  $0.3$ . The simulation results are summarized in Tables 5.1, 5.2 and 5.3 for  $\Delta = -0.1, 0.05$  and  $0.3$ .

Based on the tables we can make the following conclusions:

- 1) All the coverage probabilities converge to the nominal level as the sample increases. The coverage probabilities of the WEL-based CIs are closer to the theoretical confidence level, especially when the sample size is larger.
- 2) The proposed methods work for correlated ( $r = 0.3, r = 0.7$ ) but also for the non-correlated ( $r = 0$ ) cases. The best coverage probabilities occur when the correlation between the pair of test observations is high.

Table 5.1: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\Delta = -0.1$  at different censoring rates (CR) with 95% confidence level.

CR	$(m, n)$	$r = 0$			$r = 0.3$			$r = 0.7$		
		NA	WEL	AWEL	NA	WEL	AWEL	NA	WEL	AWEL
CR1	(50, 50)	0.912 (0.229)	0.923 (0.183)	0.931 (0.184)	0.915 (0.230)	0.924 (0.185)	0.929 (0.187)	0.923 (0.229)	0.928 (0.185)	0.930 (0.188)
	(70, 50)	0.914 (0.228)	0.925 (0.184)	0.927 (0.188)	0.924 (0.226)	0.929 (0.189)	0.931 (0.189)	0.927 (0.231)	0.929 (0.186)	0.932 (0.189)
	(70, 70)	0.920 (0.214)	0.929 (0.163)	0.930 (0.171)	0.921 (0.215)	0.928 (0.163)	0.932 (0.167)	0.922 (0.222)	0.930 (0.169)	0.936 (0.170)
	(100, 70)	0.926 (0.191)	0.931 (0.138)	0.934 (0.143)	0.928 (0.192)	0.930 (0.140)	0.936 (0.146)	0.925 (0.190)	0.930 (0.145)	0.937 (0.149)
	(100, 100)	0.932 (0.181)	0.945 (0.133)	0.948 (0.135)	0.938 (0.181)	0.945 (0.129)	0.947 (0.132)	0.939 (0.183)	0.942 (0.129)	0.944 (0.129)
	(50, 50)	0.882 (0.336)	0.899 (0.201)	0.901 (0.202)	0.881 (0.334)	0.900 (0.202)	0.908 (0.206)	0.882 (0.337)	0.902 (0.201)	0.908 (0.206)
CR2	(70, 50)	0.891 (0.338)	0.901 (0.194)	0.909 (0.198)	0.891 (0.340)	0.903 (0.191)	0.908 (0.197)	0.893 (0.339)	0.902 (0.193)	0.907 (0.199)
	(70, 70)	0.885 (0.309)	0.924 (0.177)	0.926 (0.182)	0.887 (0.308)	0.925 (0.175)	0.930 (0.179)	0.890 (0.311)	0.918 (0.179)	0.922 (0.183)
	(100, 70)	0.901 (0.278)	0.928 (0.159)	0.930 (0.163)	0.904 (0.276)	0.927 (0.158)	0.932 (0.163)	0.904 (0.279)	0.930 (0.166)	0.933 (0.168)
	(100, 100)	0.907 (0.273)	0.930 (0.154)	0.932 (0.155)	0.906 (0.277)	0.931 (0.154)	0.935 (0.159)	0.905 (0.275)	0.929 (0.154)	0.934 (0.157)
	(50, 50)	0.827 (0.381)	0.840 (0.220)	0.848 (0.224)	0.841 (0.380)	0.843 (0.221)	0.846 (0.225)	0.829 (0.383)	0.842 (0.222)	0.850 (0.226)
	(70, 50)	0.844 (0.383)	0.860 (0.222)	0.865 (0.225)	0.834 (0.384)	0.861 (0.221)	0.868 (0.227)	0.842 (0.333)	0.860 (0.225)	0.866 (0.226)
CR3	(70, 70)	0.841 (0.354)	0.859 (0.204)	0.861 (0.226)	0.844 (0.357)	0.859 (0.206)	0.868 (0.210)	0.845 (0.357)	0.860 (0.206)	0.869 (0.211)
	(100, 70)	0.875 (0.329)	0.886 (0.188)	0.889 (0.190)	0.871 (0.330)	0.888 (0.191)	0.884 (0.198)	0.870 (0.329)	0.881 (0.191)	0.887 (0.195)
	(100, 100)	0.888 (0.328)	0.909 (0.186)	0.913 (0.190)	0.905 (0.322)	0.911 (0.183)	0.912 (0.191)	0.892 (0.326)	0.919 (0.183)	0.920 (0.186)

Table 5.2: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\Delta = 0.05$  at different censoring rates (CR) with 95% confidence level.

CR	$(m, n)$	$r = 0$			$r = 0.3$			$r = 0.7$		
		NA	WEL	AWEL	NA	WEL	AWEL	NA	WEL	AWEL
CR1	(50, 50)	0.921	0.934	0.939	0.925	0.939	0.941	0.925	0.937	0.940
		(0.230)	(0.185)	(0.188)	(0.234)	(0.188)	(0.191)	(0.229)	(0.188)	(0.192)
	(70, 50)	0.920	0.937	0.939	0.923	0.936	0.939	0.926	0.939	0.942
		(0.232)	(0.186)	(0.191)	(0.231)	(0.188)	(0.188)	(0.237)	(0.190)	(0.193)
	(70, 70)	0.929	0.939	0.942	0.924	0.947	0.949	0.928	0.941	0.946
		(0.225)	(0.170)	(0.173)	(0.222)	(0.171)	(0.175)	(0.224)	(0.176)	(0.180)
(100, 70)	0.936	0.951	0.955	0.938	0.953	0.955	0.927	0.947	0.950	
	(0.194)	(0.139)	(0.148)	(0.194)	(0.141)	(0.144)	(0.194)	(0.147)	(0.150)	
(100, 100)	0.935	0.954	0.954	0.936	0.955	0.957	0.940	0.952	0.957	
	(0.185)	(0.137)	(0.141)	(0.185)	(0.130)	(0.135)	(0.184)	(0.131)	(0.136)	
CR2	(50, 50)	0.914	0.930	0.932	0.911	0.931	0.938	0.909	0.933	0.938
		(0.341)	(0.207)	(0.210)	(0.339)	(0.217)	(0.223)	(0.344)	(0.209)	(0.211)
	(70, 50)	0.910	0.932	0.940	0.915	0.935	0.939	0.914	0.934	0.938
		(0.342)	(0.200)	(0.205)	(0.344)	(0.199)	(0.201)	(0.343)	(0.200)	(0.209)
	(70, 70)	0.920	0.940	0.947	0.919	0.940	0.946	0.916	0.943	0.945
		(0.305)	(0.174)	(0.180)	(0.305)	(0.173)	(0.174)	(0.308)	(0.177)	(0.180)
(100, 70)	0.922	0.947	0.946	0.924	0.947	0.949	0.926	0.948	0.950	
	(0.279)	(0.162)	(0.169)	(0.281)	(0.165)	(0.166)	(0.276)	(0.167)	(0.171)	
(100, 100)	0.917	0.949	0.950	0.920	0.941	0.945	0.919	0.949	0.950	
	(0.277)	(0.156)	(0.158)	(0.280)	(0.159)	(0.165)	(0.277)	(0.157)	(0.160)	
CR3	(50, 50)	0.836	0.863	0.871	0.824	0.866	0.868	0.840	0.868	0.880
		(0.385)	(0.226)	(0.228)	(0.382)	(0.227)	(0.230)	(0.385)	(0.227)	(0.232)
	(70, 50)	0.845	0.888	0.895	0.838	0.881	0.888	0.839	0.887	0.889
		(0.387)	(0.226)	(0.230)	(0.388)	(0.227)	(0.228)	(0.383)	(0.226)	(0.229)
	(70, 70)	0.855	0.899	0.900	0.859	0.890	0.899	0.854	0.891	0.898
		(0.358)	(0.208)	(0.220)	(0.361)	(0.210)	(0.217)	(0.361)	(0.212)	(0.217)
(100, 70)	0.885	0.916	0.919	0.884	0.919	0.926	0.887	0.925	0.830	
	(0.333)	(0.193)	(0.199)	(0.332)	(0.196)	(0.200)	(0.335)	(0.196)	(0.199)	
(100, 100)	0.900	0.924	0.926	0.906	0.923	0.929	0.906	0.929	0.932	
	(0.331)	(0.190)	(0.196)	(0.329)	(0.187)	(0.195)	(0.328)	(0.187)	(0.190)	



Table 5.3: Comparison of coverage probabilities (average lengths) of the confidence intervals of  $\Delta = 0.3$  at different censoring rates (CR) with 95% confidence level.

CR	$(m, n)$	$r = 0$			$r = 0.3$			$r = 0.7$		
		NA	WEL	AWEL	NA	WEL	AWEL	NA	WEL	AWEL
CR1	(50, 50)	0.900	0.912	0.922	0.905	0.913	0.918	0.915	0.918	0.921
		(0.224)	(0.178)	(0.179)	(0.226)	(0.180)	(0.184)	(0.226)	(0.181)	(0.184)
	(70, 50)	0.901	0.914	0.918	0.913	0.916	0.918	0.917	0.919	0.922
		(0.225)	(0.180)	(0.183)	(0.226)	(0.181)	(0.181)	(0.229)	(0.182)	(0.183)
	(70, 70)	0.909	0.919	0.921	0.911	0.917	0.919	0.918	0.919	0.926
		(0.210)	(0.158)	(0.159)	(0.210)	(0.161)	(0.162)	(0.217)	(0.165)	(0.167)
(100, 70)	0.917	0.921	0.927	0.918	0.920	0.926	0.914	0.921	0.927	
	(0.187)	(0.133)	(0.140)	(0.184)	(0.134)	(0.144)	(0.188)	(0.141)	(0.144)	
(100, 100)	0.925	0.931	0.934	0.928	0.936	0.938	0.929	0.931	0.936	
	(0.177)	(0.128)	(0.130)	(0.178)	(0.124)	(0.129)	(0.179)	(0.124)	(0.126)	
CR2	(50, 50)	0.882	0.899	0.901	0.881	0.900	0.908	0.882	0.902	0.908
		(0.336)	(0.201)	(0.202)	(0.334)	(0.202)	(0.206)	(0.337)	(0.201)	(0.206)
	(70, 50)	0.891	0.901	0.909	0.891	0.903	0.908	0.893	0.902	0.907
		(0.338)	(0.194)	(0.198)	(0.340)	(0.191)	(0.197)	(0.339)	(0.193)	(0.199)
	(70, 70)	0.880	0.912	0.917	0.883	0.910	0.917	0.886	0.913	0.915
		(0.305)	(0.174)	(0.180)	(0.305)	(0.173)	(0.174)	(0.308)	(0.177)	(0.180)
(100, 70)	0.892	0.917	0.920	0.894	0.918	0.922	0.896	0.919	0.920	
	(0.273)	(0.154)	(0.159)	(0.277)	(0.155)	(0.162)	(0.279)	(0.159)	(0.161)	
(100, 100)	0.895	0.919	0.921	0.900	0.920	0.925	0.899	0.919	0.924	
	(0.270)	(0.150)	(0.153)	(0.272)	(0.151)	(0.155)	(0.271)	(0.151)	(0.154)	
CR3	(50, 50)	0.818	0.832	0.839	0.811	0.833	0.838	0.819	0.832	0.839
		(0.378)	(0.218)	(0.220)	(0.376)	(0.218)	(0.220)	(0.380)	(0.218)	(0.220)
	(70, 50)	0.831	0.851	0.855	0.821	0.850	0.858	0.830	0.852	0.859
		(0.380)	(0.219)	(0.221)	(0.379)	(0.220)	(0.222)	(0.378)	(0.220)	(0.221)
	(70, 70)	0.832	0.848	0.851	0.834	0.849	0.859	0.833	0.850	0.858
		(0.351)	(0.200)	(0.221)	(0.354)	(0.202)	(0.207)	(0.354)	(0.202)	(0.208)
(100, 70)	0.864	0.876	0.881	0.860	0.879	0.880	0.867	0.879	0.881	
	(0.326)	(0.184)	(0.188)	(0.325)	(0.188)	(0.189)	(0.327)	(0.187)	(0.189)	
(100, 100)	0.878	0.899	0.900	0.886	0.900	0.902	0.888	0.909	0.903	
	(0.325)	(0.183)	(0.186)	(0.321)	(0.179)	(0.190)	(0.322)	(0.180)	(0.182)	

- 3) The lengths of the CIs decrease with the larger sample size and increase with the heavy censoring rate. AWEL intervals are wider than WEL's. In addition, WEL and AWEL CIs have shorter average length than NA CIs.
- 4) AWEL boosted the coverage accuracy for the different combinations of light (10%), moderate (25%), and heavy (40%) censoring rate considered. In all cases, the AWEL compensates for the loss of information due to the censoring.

## 5.4 Real Applications

This section applies the proposed methods to compare two regression models. This is done by comparing the AUCs generated by risk scores induced by the two models. To compare the AUCs we will compute the CI for their difference. We reuse here the PBC data from Section 4.4 in Chapter 4. The study is a double-blinded randomized trial in the liver's primary biliary cirrhosis, a rare autoimmune liver disease, comparing the drug D-penicillamine (DPCA) with a placebo. The DPCA group has 158 patients with a censoring rate of 52%, and the placebo group 154 with a 55% censoring rate. Different covariates values were recorded during the study. Bilirubin is one of the covariates that has shown to be the strongest univariate predictor of survival (Fleming and Harrington, 1991). Often statistical models are built to assess the influence of those covariates on the disease outcome. Statistical scores are calculated based on the models and are used for the comparison. These scores play the same role as markers play in diagnostics tests. We consider the Cox regression model with five covariates as:  $\log(\text{bilirubin})$ , albumin,  $\log(\text{prothrombin time})$ , edema, and age. The second model contains all the previous covariates except the  $\log(\text{bilirubin})$ . The models are denoted model 1 and model 2, respectively. Prognostic scores have been created based on the two models, and they showed good discrimination power. These two models have been considered by Fleming and Harrington (1991) to show the effect of the bilirubin on patients survival. The scores are based on the estimated regression coefficients (see Fleming and Harrington, 1991, Section 4.4 for more details). Heagerty and Zheng (2005) also used

the same scores for their time-dependent ROC curves. The scores are also highly correlated with a Pearson correlation of 0.84. We compute the CI for the difference between the AUCs and the lengths of the CI based on the two model scores using the NA, WEL, and AWEL methods. The difference  $\Delta$  is 0.08 and the results are summarized in Table 5.4. Figure 5.1 depicts the comparison of two AUCs for the model 1 and model 2 with the PBC data.

Table 5.4: 95% Confidence intervals for  $\Delta$  for the PBC data

Methods	Confidence interval	Length
NA	(-0.067, 0.227)	0.294
WEL	(0.026, 0.216)	0.190
AWEL	(0.023, 0.221)	0.198

The application confirms the simulation results. NA intervals are longer than these of WEL and AWEL. While the NA CI contains 0 and suggests that model 1 (with the log(Bilirubin)) and model 2 (without the log(Bilirubin)) yield the same conclusion, the WEL and AWEL CIs do not contain 0, meaning that the model 1 performs better than model 2. The WEL and AWEL CIs aligned with previous studies' findings in Fleming and Harrington (1991) and Heagerty and Zheng (2005).

## 5.5 Conclusions

This chapter considered the WEL confidence intervals for the difference between two areas under two correlated ROC curves with right-censored data. The weighted empirical log-likelihood ratio converges to a scaled chi-squared distribution. One calibration method, namely AWEL, has been applied to the proposed approach to enhance the small samples' coverage accuracy. Using the proposed methods, a confidence interval is then constructed for the difference by extensive simulations. Not only do the confidence intervals tend to the nominal level when the sample size increases for all proposed methods, but also their performance is acceptable in terms of coverage probability. The AWEL performs well when

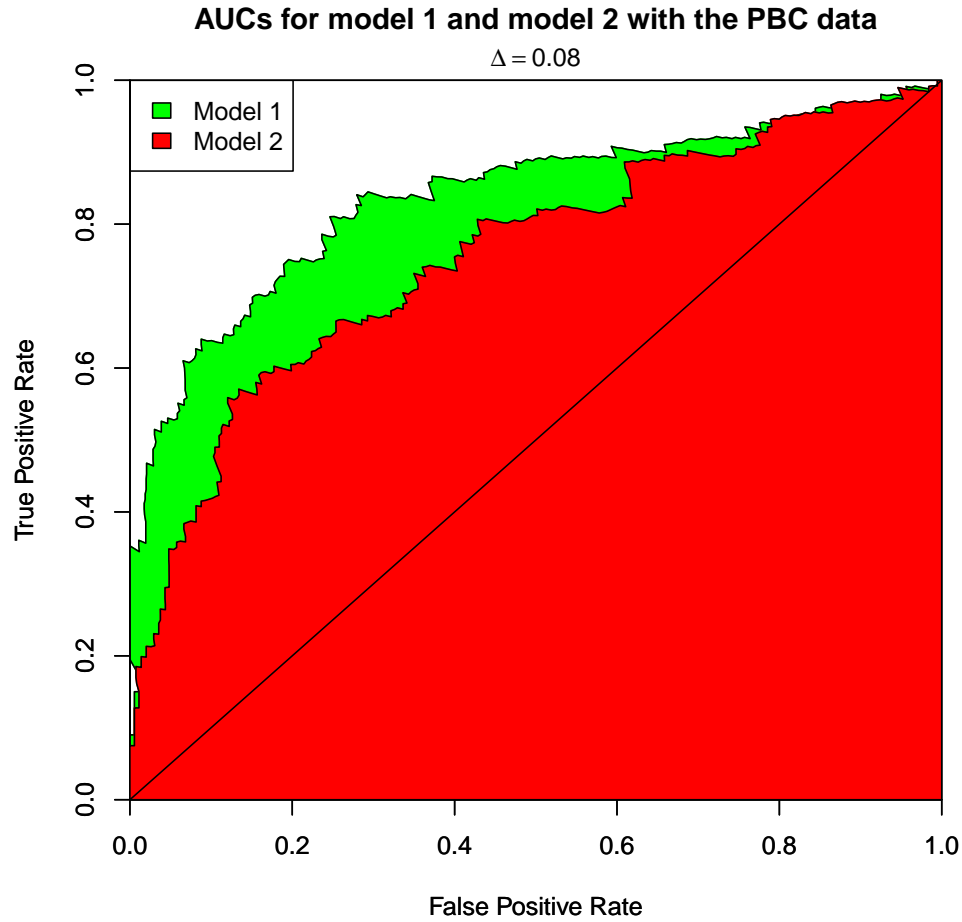


Figure 5.1: Comparison of AUCs for the PBC data set.

the censoring rate is heavy at the cost of having a longer average length of CIs. It is also easy to implement the proposed methods using the existing R packages. Finally, a real application is given to illustrate the performance of the proposed methods.

## CHAPTER 6

### DISCUSSIONS AND FUTURE WORKS

This dissertation research projects fall within the scope of developing new and appealing statistical methods to solve problems, in particular two-sample ones, related to survival data. EL (Owen 1988, 1990, 2001) is a reliable nonparametric method with the advantages of the traditional likelihood method and nonparametric methods' flexibility. The main focus of this dissertation is to extend the EL method to make it work in situations where it usually fails. Among our numerous contributions, we propose the JEL, a fast way to minimize cost in comparing two Gini indices, the NEL a combination of EL with influence functions to handle length-bias with the MRL case where other EL methods fail. The IID-EL extends the NEL approach for censored two-samples problems for the mean. We develop the WEL to compare two correlated AUCs when censoring is present, knowing that no methods exist to handle the censored case.

Throughout the dissertation, we demonstrate that the proposed methods have some advantages over the existing methods, including better coverage probability, less computation, weak regularity conditions, etc. They also have practical value, as shown by real data applications. Even though these methods were meant for a particular parameter of interest, we intend to generalize them to a broader range of statistical parameters.

For the proposed JEL in Chapter 2, we will investigate cases where the data is subject to censoring in the future. Further, adapt the method to other parameters that can be expressed as a ratio or combination of  $U$ -statistics. Due to its close relation to the Gini index ( $GINI = 2AUC - 1$ ), the AUC application is an ongoing project. It will be interesting to generalize the NEL (Chapter 3) meant for the mean residual life function with length-biased and right-censored data to other statistics functionals of the mean under the same conditions. In Chapter 4, we propose inference on the mean difference for right-censored data

using a combination of EL and i.i.d. representation technique (IID-EL). In the future, it will be interesting to extend this method to the mean difference with the data subject to length-bias and also to other parameters of interest such as quantile, distribution functions, etc. To derive a WEL (Chapter 5) for all parameters of which consistent estimators can be expressed as a weighted average of given observations is one of the immediate projects.

The independent random censoring case (failure time and censoring time are independent) has been considered all along with the study. We will extend the proposed methods to dependent censoring for all the censored cases as in some studies, covariates might be associated with both lifetime and censoring mechanisms, inducing dependent censoring. In this case, standard survival techniques, like Kaplan–Meier estimator, cannot be used or will lead to biased results.

The area of Biostatistics is inexhaustible, and through this dissertation, we intend to make the contribution to solving current and future challenges.

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## Appendix A

### PROOFS OF CHAPTER 2

The following lemmas are needed for the proofs of the theorems.

**Lemma A.1.** *Assume  $EX^2 < \infty$ . Then  $Eh^2(X_1, X_2; G) < \infty$ , for  $0 \leq G \leq 1$ .*

*Proof.*

$$\begin{aligned} Eh^2(X_1, X_2; G) &= E[(X_1 + X_2)G - |X_1 - X_2|]^2 \\ &\leq 2E\{(X_1 + X_2)G\}^2 + 2E|X_1 - X_2|^2 \end{aligned}$$

as  $E(A + B)^2 \leq 2EA^2 + 2EB^2$ .

By Cauchy-Schwarz inequality, we have

$$\begin{aligned} E\{(X_1 + X_2)G\}^2 &\leq E(X_1 + X_2)^2 G^2 \\ &\leq 2(EX_1^2 + EX_2^2)G^2 < \infty \end{aligned}$$

and  $E|X_1 - X_2|^2 \leq 2(EX_1^2 + EX_2^2)G^2 < \infty$ . Thus,  $Eh^2(X_1, X_2; G) < \infty$ .

□

**Lemma A.2.** *Assume  $EX_1^2 < \infty$  and  $\sigma_g^2(\Delta, G_2) > 0$ . Then, for each  $\Delta$ , as  $n \rightarrow \infty$ , we have*

$$P(\min_{1 \leq i \leq n} \widehat{V}_i(\Delta) < 0 < \max_{1 \leq i \leq n} \widehat{V}_i(\Delta)) \rightarrow 1,$$

where  $\widehat{V}_i(\Delta)$  is given by equation (2.5).

*Proof.* Define  $\omega(x, \Delta + \widehat{G}_2) = (\Delta + \widehat{G}_2)Eh_2(x) - Eh_1(x, X_1)$ ,

$\psi(x_1, x_2; \Delta + \widehat{G}_2) = h(x_1, x_2; \Delta + \widehat{G}_2) - \omega(x_1; \Delta + \widehat{G}_2) - \omega(x_2; \Delta + \widehat{G}_2)$ .

By the Hoeffding decomposition, we have

$$M_n(\Delta) = \frac{1}{2n} \sum_{i=1}^n \omega(X_i; \Delta + \widehat{G}_2) + \binom{n}{2}^{-1} \sum_{i < j}^n \psi(X_i, X_j; \Delta + \widehat{G}_2).$$

After some simple algebra, one can write

$$\begin{aligned} \widehat{V}_i(\Delta) &= 2\omega(X_i; \Delta + \widehat{G}_2) + \frac{2}{n-2} \sum_{k=i, k \neq i} \psi(X_i, X_k; \Delta + \widehat{G}_2) - \\ &\quad \binom{n-1}{2}^{-1} \sum_{i < j}^n \psi(X_i, X_j; \Delta + \widehat{G}_2) \\ &= 2\omega(X_i, \Delta + \widehat{G}_2) + (\widehat{G}_2 - G_2)h_2(X_i) + \\ &\quad \frac{2}{n-2} \sum_{k=i, k \neq i} \psi(X_i, X_k; \Delta + G_2) - \binom{n-1}{2}^{-1} \sum_{i < j}^n \psi(X_i, X_j; \Delta + \widehat{G}_2) \\ &:= 2\omega(X_i, \Delta + \widehat{G}_2) + (\widehat{G}_2 - G_2)h_2(X_i) + R_{ni}(\Delta + \widehat{G}_2). \end{aligned}$$

We have

$$E[(\widehat{G}_2 - G_2)h_2(X_i)]^2 \rightarrow 0, \quad \text{as } n \rightarrow \infty.$$

Since  $\widehat{G}_2 - G_2 = O_p(n^{-1/2})$  by asymptotic normality of  $\widehat{G}_2$  (Hoeffding 1948) and  $\max_{1 \leq i \leq n} |h_2(X_i)| = o_p(n^{1/2})$  by Lemma A.4 of Jing et al. (2009). Further, by Lemma A.1,

$$E[\psi(X_1, X_2; \Delta + \widehat{G}_2)]^2 = E[h(X_1, X_2; \Delta + \widehat{G}_2) - \omega(X_1, \Delta + \widehat{G}_2) - \omega(X_2, \Delta + \widehat{G}_2)]^2 < \infty.$$

Then

$$E[R_{ni}^2(\Delta + \widehat{G}_2)] \leq Cn^{-1}E[\psi(X_1, X_2; \Delta + \widehat{G}_2)]^2 + Cn^{-2}E[\psi(X_1, X_2; \Delta + \widehat{G}_2)]^2 \rightarrow 0 \text{ as } n \rightarrow \infty,$$

for some constant C. Therefore,  $R_{ni}^2(\Delta + \widehat{G}_2) \rightarrow 0$  and  $\widehat{V}_i(\Delta) \rightarrow 2\omega(X_i, \Delta + \widehat{G}_2)$  in probability.

Thus, using the same argument as in the proof of Lemma A.1 in Jing et al. (2009), as  $n \rightarrow \infty$ ,

$$P\left(\min_{1 \leq i \leq n} \widehat{V}_i(\Delta) < 0 < \max_{1 \leq i \leq n} \widehat{V}_i(\Delta)\right) \rightarrow 1,$$

for every  $\Delta$ .

□

**Lemma A.3.** Assume  $EX_1^2 < \infty$ . Then we have

$$\frac{\sqrt{n}M_n(\Delta)}{2\sigma_g(\Delta, G_2)} \xrightarrow{\mathcal{D}} N(0, 1) \text{ as } n \rightarrow \infty,$$

where  $M_n(\Delta)$  is given by equation (2.4).

*Proof.* Let

$$M_n^o(\Delta) = \binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h(X_i, X_j; \Delta + G_2),$$

$$M_n(\Delta) = [M_n(\Delta) - M_n^o(\Delta)] + M_n^o(\Delta).$$

It is clear that  $M_n^o(\Delta)$  is a  $U$ -statistic for the true values  $\Delta$  and  $G_2$ , and

$$\frac{\sqrt{n}M_n^o(\Delta)}{2\sigma_g(\Delta, G_2)} \xrightarrow{\mathcal{D}} N(0, 1)$$

as  $n \rightarrow \infty$  according to Lemma A.2 of Jing et al. (2009). Furthermore,

$$M_n(\Delta) - M_n^o(\Delta) = (\widehat{G}_2 - G_2) \binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h_2(X_i),$$

which is negligible since  $\widehat{G}_2 - G_2 = o_p(1)$ .

□

**Lemma A.4.** Let  $S_n(\Delta) = n^{-1} \sum_{i=1}^n [\widehat{V}_i(\Delta)]^2$ . Assume  $EX_1^2 < \infty$ . Then with probability one, we have

$$S_n(\Delta) = 4\sigma_g^2(\Delta, G_2) + o_p(1),$$

where  $\widehat{V}_i(\Delta)$  is given by equation (2.5).

*Proof.* Let  $\widehat{V}_i^o(\Delta)$  be the value of  $\widehat{V}_i(\Delta)$  for the true value  $G_2$ . Define  $S_n^o(\Delta) = n^{-1} \sum_{i=1}^n [\widehat{V}_i^o(\Delta)]^2$ .

We have

$$S_n(\Delta) = S_n^o(\Delta) + (S_n(\Delta) - S_n^o(\Delta)).$$

From Lemma A.3 in Jing et al. (2009),  $S_n^o(\Delta) = 4\sigma_g^2(\Delta, G_2) + o_p(1)$ . It suffices to show that  $S_n(\Delta) - S_n^o(\Delta)$  is negligible. In fact, we can rewrite  $M_n(\Delta)$  as

$$\begin{aligned} M_n(\Delta) &= (\Delta + \widehat{G}_2) \binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h_2(X_i) - \binom{n}{2}^{-1} \sum_{1 \leq i < j \leq n} h_1(X_i, X_j) \\ &:= (\Delta + \widehat{G}_2) U_n^2 - U_n^1, \end{aligned}$$

where  $U_n^i$ ,  $i = 1, 2$ , are  $U$ -statistics with kernels being  $h_i(\cdot)$ ,  $i = 1, 2$ , respectively. Define

$$\widehat{V}_i^1 = nU_n^1 - (n-1)U_{n-1}^{1(-i)} \quad \text{and} \quad \widehat{V}_i^2 = nU_n^2 - (n-1)U_{n-1}^{2(-i)}.$$

Then

$$\widehat{V}_i(\Delta) = (\Delta + \widehat{G}_2)\widehat{V}_i^2 - \widehat{V}_i^1,$$

and thus

$$\begin{aligned} S_n(\Delta) - S_n^o(\Delta) &= \frac{1}{n} \sum_{i=1}^n \{[\widehat{V}_i(\Delta)]^2 - [\widehat{V}_i^o(\Delta)]^2\} \\ &= (\widehat{G}_2 - G_2)(2\Delta + \widehat{G}_2 + G_2) \frac{1}{n} \sum_{i=1}^n [\widehat{V}_i^1]^2 - 2(\widehat{G}_2 - G_2) \frac{1}{n} \sum_{i=1}^n [\widehat{V}_i^1 \widehat{V}_i^2] \\ &\rightarrow 0, \quad \text{as } n \rightarrow \infty. \end{aligned}$$

The above result is valid because

$$\begin{aligned} \frac{1}{n} \sum_{i=1}^n [V_i^1]^2 &= \frac{1}{n} \sum_{i=1}^n (\widehat{V}_i^1 - EU_n^1 + EU_n^1)^2 \\ &= \frac{1}{n} \sum_{i=1}^n (\widehat{V}_i^1 - EU_n^1)^2 + 2EU_n^1 \frac{1}{n} \sum_{i=1}^n \widehat{V}_i^1 - (EU_n^1)^2 \\ &= \frac{1}{n} \sum_{i=1}^n (\widehat{V}_i^1 - EU_n^1)^2 + 2(EU_n^1) U_n^1 - (EU_n^1)^2, \end{aligned}$$



in which  $n^{-1} \sum_{i=1}^n (\widehat{V}_i^1 - EU_n^1)^2 = C + o_p(1)$ , for some constant  $C$  by Lemma A.3 of Jing et al. (2009), and  $2U_n^1 EU_n^1 - (EU_n^1)^2$  goes to a finite number. That is,  $n^{-1} \sum_{i=1}^n [\widehat{V}_i^1]^2 = C + o_p(1)$  for some constant  $C$ . We can establish a similar result for  $n^{-1} \sum_{i=1}^n [\widehat{V}_i^2]^2$  and therefore  $n^{-1} \sum_{i=1}^n [\widehat{V}_i^1 \widehat{V}_i^2] < \infty$ . In addition,  $\widehat{G}_2 - G_2 = O_p(n^{-1/2})$ . Thus,  $S_n(\Delta) - S_n^o(\Delta) \rightarrow 0$ .

□

**Lemma A.5.** Let  $H_n(\Delta, \widehat{G}_2) = \max_{1 \leq i \neq j \leq n} |h(X_i, X_j; \Delta + \widehat{G}_2)|$ . Assume  $EX_1^2 < \infty$ . Then  $H_n(\Delta, \widehat{G}_2) = o_p(n^{1/2})$ , with probability one.

*Proof.*

$$\begin{aligned} H_n(\Delta, \widehat{G}_2) &= \max_{1 \leq i \neq j \leq n} |(\Delta + \widehat{G}_2)h_2(X_i, X_j) - h_1(X_i)| \\ &\leq 2 \max_{1 \leq i \neq j \leq n} |h_2(X_i, X_j)| + \max_{1 \leq i \leq n} |h_1(X_i)| \\ &= o_p(n^{1/2}) + o_p(n^{1/2}) \\ &= o_p(n^{1/2}), \end{aligned}$$

by applying Lemma A.4 of Jing et al. (2009) to the functions  $h_2$  and  $h_1$ .

□

*Proof of Theorem 2.2.1.* Lemma A.2 guarantees the existence and uniqueness of  $\lambda$ , solution for equation (2.8). Following the above lemmas, and using the same arguments as in the proof of Theorem 1 in Jing et al. (2009), we have  $|\lambda(\Delta)| = O_p(n^{-1/2})$  and also

$$\begin{aligned} \lambda(\Delta) &= S_n^{-1}(\Delta) \frac{1}{n} \sum_{i=1}^n [\widehat{V}_i(\Delta)] + \beta \\ &= S_n^{-1}(\Delta) M_n(\Delta) + \beta, \end{aligned}$$

where  $|\beta| = o_p(n^{-1/2})$ . Then, we can write

$$-2 \log R(\Delta) = \frac{nM_n^2(\Delta)}{S_n(\Delta)} - nS_n(\Delta)\beta^2 + 2 \sum_{i=1}^n \eta_i. \quad (\text{A.1})$$

In equation (A.1),  $nM_n^2(\Delta)/S_n(\Delta) \xrightarrow{\mathcal{D}} \chi_1^2$  by Lemmas A.3 and A.4,

$$|-nS_n(\Delta)\beta^2| = n(4\sigma_g^2(\Delta, G_2) + o_p(1))o_p(n^{-1}) = o_p(1),$$

and

$$\left| \sum_{i=1}^n \eta_i \right| \leq C|\lambda(\Delta)|^3 \sum_{i=1}^n |\widehat{V}_i(\Delta)|^3 = O_p(n^{-3/2}) o_p(n^{3/2}) = o_p(1),$$

for some positive constant  $C$ .

Therefore,  $-2 \log R(\Delta) \xrightarrow{\mathcal{D}} \chi_1^2$  by Slutsky's theorem. □

*Proof of Theorem 2.2.2.* The proof of theorem 2.2.2 is essentially based on above lemmas with slight adjustments as applied by Chen et al. (2008). Hence, we sketch it here.

We first, show that  $|\lambda(\Delta)| = O_p(n^{-1/2})$ . From equation (2.9), we can write

$$\begin{aligned} 0 &= \frac{1}{n} \left| \sum_{i=1}^{n+1} \widehat{V}_i(\Delta) - \lambda(\Delta) \sum_{i=1}^{n+1} \frac{\widehat{V}_i^2(\Delta)}{1 + \lambda(\Delta)\widehat{V}_i(\Delta)} \right| \\ &\geq \frac{|\lambda(\Delta)|}{n} \sum_{i=1}^{n+1} \frac{\widehat{V}_i^2(\Delta)}{1 + \lambda(\Delta)\widehat{V}_i(\Delta)} - \frac{1}{n} \sum_{i=1}^{n+1} \widehat{V}_i(\Delta) \\ &\geq \frac{|\lambda(\Delta)|S_n(\Delta)}{1 + |\lambda(\Delta)|W_n(\Delta)} - \left| \frac{1}{n} \sum_{i=1}^n \widehat{V}_i(\Delta) \right| \left(1 - \frac{a_n}{n}\right), \end{aligned}$$

where  $S_n(\Delta) = n^{-1} \sum_{i=1}^n [\widehat{V}_i(\Delta)]^2$  and  $W_n(\Delta) = \max_{1 \leq i \leq n} |\widehat{V}_i(\Delta)|$ . By Lemmas A.3, A.4, and A.5, one has  $n^{-1} \sum_{i=1}^n [\widehat{V}_i(\Delta)] = M_n(\Delta) = O_p(n^{-1/2})$ ,  $S_n(\Delta) = 4\sigma_g^2(\Delta, G_2) + o_p(1)$  and  $W_n(\Delta) = o_p(n^{1/2})$ . Combined with  $a_n = o_p(n)$ , we get  $|\lambda(\Delta)| = O_p(n^{-1/2})$ .

Next, we show that  $\lambda(\Delta) = S_n^{-1}(\Delta)M_n(\Delta) + o_p(n^{-1/2})$ . From equation (2.9), we can write

$$\begin{aligned} 0 &= \frac{1}{n} \sum_{i=1}^{n+1} \frac{\widehat{V}_i(\Delta)}{1 + \lambda(\Delta)\widehat{V}_i(\Delta)} \\ &= \frac{1}{n} \sum_{i=1}^{n+1} \widehat{V}_i(\Delta) - \lambda(\Delta)S_n(\Delta) + o_p(n^{-1/2}), \end{aligned}$$

by noting that the  $(n+1)th$  term of the summation is  $a_n O_p(n^{-3/2})$ .

Hence, when  $n \rightarrow \infty$ ,  $\lambda(\Delta) = S_n^{-1}(\Delta)M_n(\Delta) + o_p(n^{-1/2})$ .

Finally, expanding  $-2 \log R^A(\Delta)$  and replacing  $\lambda(\Delta)$  by its expansion, one has that

$$\begin{aligned} -2 \log R^A(\Delta) &= 2 \sum_{i=1}^{n+1} \log(1 + \lambda(\Delta)\widehat{V}_i(\Delta)) \\ &= 2 \sum_{i=1}^{n+1} \left( \lambda(\Delta)\widehat{V}_i(\Delta) - \lambda(\Delta)^2\widehat{V}_i^2(\Delta)/2 \right) + o_p(1) \\ &= \frac{nM_n^2(\Delta)}{S_n(\Delta)} + o_p(1). \end{aligned}$$

Thus,  $-2 \log R^A(\Delta)$  converges to  $\chi_1^2$  by Lemmas A.3 and A.4 and Slutsky's theorem.

□

## Appendix B

### PROOFS OF CHAPTER 3

Let us assume the following regularity conditions:

- 1)  $F$  and  $G$  are continuous,
- 2)  $\int_0^\infty \frac{t^4}{1-G(t)} dF(t) < \infty$ ,
- 3)  $P(C > \tau_F) > 0$ .

Assumption 1 is natural because the time variable is continuous. Assumption 2 ensures that the variance of  $W_i(m)$  is finite. Assumption 3 states that the support of  $C$  covers the support of  $T$ . Therefore, one can estimate the MRL at every point. The following lemmas will be needed for the proof of the theorems.

**Lemma B.1.** *Assume that the regularity conditions hold. Then, as  $n \rightarrow \infty$ , we have*

$$\frac{1}{\sqrt{n}} \sum_{i=1}^n W_{ni}(m_0) \rightarrow N(0, \sigma^2) \quad \text{in distribution,}$$

where  $\sigma^2 = \text{Var}(W_i(m_0))$ , and  $W_i(m)$ ,  $W_{ni}(m)$  are given by Equation (3.2) and Equation (3.3), respectively.

*Proof.* Recall the martingale property of  $d\hat{\Lambda}^C$  :

$$\sum_{i=1}^n \{dI(Z_i \leq s, \delta_i = 0) - I(Z_i \geq s) d\hat{\Lambda}^C(s)\} = 0.$$

We can easily show that

$$\begin{aligned}
\frac{1}{\sqrt{n}} \sum_{i=1}^n W_{ni}(m_0) &= \frac{1}{\sqrt{n}} U(m_0) + \sqrt{n} \int_0^\infty \frac{\sum_{j=1}^n \omega_{(j)} I(Z_{(j)} \geq s) \Phi(Z_{(j)}, m_0)}{\sum_{j=1}^n I(Z_j \geq s)} \\
&\quad \times \sum_{i=1}^n \{dI(Z_i \leq s, \delta_i = 0) - I(Z_i \geq s) d\widehat{\Lambda}^C(s)\} \\
&= \frac{1}{\sqrt{n}} U(m_0).
\end{aligned}$$

Thus by the proof of Lemma 5.1 in the Appendix of Liang et al. (2016), Lemma B.1 is valid.  $\square$

**Lemma B.2.** *Assume that the regularity conditions hold. Then, as  $n \rightarrow \infty$ , we have*

$$\frac{1}{n} \sum_{i=1}^n W_{ni}^2(m_0) \rightarrow \sigma^2 \quad \text{in probability,}$$

where  $W_{ni}(m)$  is given by Equation (3.3).

*Proof.* For each  $i$ , it can be shown that

$$\begin{aligned}
W_{ni}(m_0) &= W_i(m_0) + \frac{\Phi(Z_i, m_0) \delta_i}{1 - \widehat{G}_n(Z_i)} - \frac{\Phi(Z_i, m_0) \delta_i}{1 - G(Z_i)} \\
&+ \int_0^\infty \left[ \frac{\sum_{j=1}^n \omega_{(j)} I(Z_{(j)} \geq s) \Phi(Z_{(j)}, m_0)}{n^{-1} \sum_{j=1}^n I(Z_j \geq s)} - \frac{\psi(s, m_0)}{\overline{H}(s)} \right] [dN_i^C(s) - I(Z_i \geq s) d\Lambda^C(s)] \\
&- \int_0^\infty \frac{\sum_{j=1}^n \omega_{(j)} I(Z_{(j)} \geq s) \Phi(Z_{(j)}, m_0)}{n^{-1} \sum_{j=1}^n I(Z_j \geq s)} I(Z_i \geq s) d[\widehat{\Lambda}^C(s) - \Lambda^C(s)] \\
&:= W_i(m_0) + r_{i1} + r_{i2} + r_{i3}.
\end{aligned}$$

By the consistency of the Kaplan-Meier estimator  $\widehat{G}_n$ ,

$\sum_{j=1}^n \omega_{(j)} I(Z_{(j)} \geq s) \Phi(Z_{(j)}, m_0)$ , and  $n^{-1} \sum_{j=1}^n I(Z_j \geq s)$ , we have

$$|r_{i1}| \leq O_p(1) \sup_{s \leq Z_{(n)}} |\widehat{G}_n(s) - G(s)| \left( 1 + \sup_{s \leq Z_{(n)}} \left| \frac{\widehat{G}_n(s) - G(s)}{1 - \widehat{G}_n(s)} \right| \right) = o_p(1),$$

as Zhou (1991) proved that  $\sup_{s \leq Z_{(n)}} \left| \frac{\widehat{G}_n(s) - G(s)}{1 - \widehat{G}_n(s)} \right|$  is bounded in probability,

$$|r_{i2}| \leq O_p(1) \sup_{s \leq Z_{(n)}} \left| \frac{\sum_{j=1}^n \omega_{(j)} I(Z_{(j)} \geq s) \Phi(Z_j, m_0)}{n^{-1} \sum_{j=1}^n I(Z_j \geq s)} - \frac{\psi(s, m_0)}{\overline{H}(s)} \right| = o_p(1),$$

and noting that

$$\widehat{\Lambda}^C(s) - \Lambda^C(s) = \int_0^s \frac{dM^C(u)}{Y(u)},$$

where  $M^C(u) = N^C(u) - \int_0^u Y(t) d\Lambda^C(t)$  is a martingale,

$$r_{i3} = \int_0^\infty \frac{g_n(s)}{Y(s)} d[\widehat{\Lambda}^C(s) - \Lambda^C(s)],$$

where  $g_n(s) = nI(Z_i \geq s) \sum_{j=1}^n \omega_{(j)} I(Z_{(j)} \geq s) \Phi(Z_{(j)}, m_0)$ , and  $g_n(s)/Y(s)$  predictable and locally bounded, is a martingale integral. Following the lines of Andersen et al. (1993, p.190), we apply Lengart's inequality to  $r_{i3}$  and have, for any  $\varepsilon, \delta > 0$

$$P\left(\sup_{s \leq Z_{(n)}} |r_{i3}| > \varepsilon\right) \leq \frac{\delta}{\varepsilon^2} + P\left(\int_0^{Z_{(n)}} \frac{g_n(s)}{Y(s)} d\Lambda^C(s) > \delta\right) \rightarrow 0.$$

Then,  $r_{i3}$  converges to zero uniformly in probability for  $s \leq Z_{(n)}$ . Therefore

$$|r_{i3}| = o_p(1).$$

We can write

$$\begin{aligned} & \left| \frac{1}{n} \sum_{i=1}^n W_{ni}^2(m_0) - \frac{1}{n} \sum_{i=1}^n W_i^2(m_0) \right| \\ &= \left| \frac{1}{n} \sum_{i=1}^n (W_{ni}(m_0) - W_i(m_0)) (W_{ni}(m_0) - W_i(m_0) + 2W_i(m_0)) \right| \\ &\leq \frac{1}{n} \sum_{i=1}^n (W_{ni}(m_0) - W_i(m_0))^2 + \left| \frac{2}{n} \sum_{i=1}^n (W_{ni}(m_0) - W_i(m_0)) W_i(m_0) \right| \\ &:= I_1 + I_2. \end{aligned}$$

From the order  $o_p(1)$  of  $r_{i1}$ ,  $r_{i2}$  and  $r_{i3}$ ,

$$W_{ni}(m_0) - W_i(m_0) = r_{i1} + r_{i2} + r_{i3} = o_p(1).$$

It can be easily shown that  $I_1 = o_p(1)$  and  $I_2 = o_p(1)$ . Thus,

$$\frac{1}{n} \sum_{i=1}^n W_{ni}^2(m_0) = \frac{1}{n} \sum_{i=1}^n W_i^2(m_0) + o_p(1).$$

By the law of large numbers,

$$\frac{1}{n} \sum_{i=1}^n W_{ni}^2(m_0) \rightarrow \sigma^2,$$

in probability as  $n \rightarrow \infty$ . □

*Proof of Theorem 3.2.1.* Following Alemjdjrodo and Zhao (2019), we prove it. First we need to show that (i)  $\max_{1 \leq i \leq n} |W_{ni}(m_0)| = o_p(n^{1/2})$  and (ii)  $\lambda = O_p(n^{-1/2})$ , where  $\lambda$  is a solution

of Equation (3.4). For each  $i$ ,

$$W_{ni}(m_0) = W_i(m_0) + r_{i1} + r_{i2} + r_{i3}.$$

Since  $W_i(m_0)$ ,  $i = 1, \dots, n$  are i.i.d. random variables with finite second moment, by Lemma 11.2 of Owen (2001),  $\max_{1 \leq i \leq n} |W_{ni}(m_0)| = o_p(n^{1/2})$ . Note  $r_{i1}$ ,  $r_{i2}$  and  $r_{i3}$  are of order  $o_p(1)$ , by applying similar argument in the proof of Theorem 2.2 of Owen (2001), we can prove that  $\lambda = O_p(n^{-1/2})$ . We can easily derive

$$\lambda = \frac{\frac{1}{n} \sum_{i=1}^n W_{ni}(m_0)}{\frac{1}{n} \sum_{i=1}^n W_{ni}^2(m_0)} + o_p(n^{-1/2}).$$

Therefore, as  $n \rightarrow \infty$

$$\begin{aligned} l(m_0) &= \sum_{i=1}^n \lambda W_{ni}(m_0) + o_p(1) \\ &= \frac{\left( \sum_{i=1}^n W_{ni}(m_0) \right)^2}{\sum_{i=1}^n W_{ni}^2(m_0)} + o_p(1) \\ &= \left( \frac{\frac{1}{\sqrt{n}} \sum_{i=1}^n W_{ni}(m_0)}{\sqrt{\sigma^2 + o_p(1)}} \right)^2 + o_p(1) \\ &\xrightarrow{\mathcal{D}} \chi_1^2. \end{aligned}$$

□

*Proof of Theorem 3.2.2.* Let  $S_n^2(m_0) = n^{-1} \sum_{i=1}^n W_{ni}^2(m_0)$  and  $W_n^*(m_0) = \max_{1 \leq i \leq n} |W_{ni}(m_0)|$ . By Lemma B.1, one has  $\bar{W}_n(m_0) = n^{-1} \sum_{i=1}^n W_{ni}(m_0) = O_p(n^{-1/2})$ . By Lemma B.2,  $S_n^2(m_0) = \sigma^2 + o_p(1)$  and by the result (i) in the proof of Theorem 3.2.1, we have  $W_n^*(m_0) = o_p(n^{1/2})$ . Using these results, we prove that  $|\lambda^A| = O_p(n^{-1/2})$  as Zhao et al. (2015) and Wang and



Zhao (2016) did. Next, from Equation (3.5), we have  $\lambda^A = \overline{W}_n(m_0)/S_n^2(m_0) + o_p(n^{-1/2})$ .

Finally, we have

$$\begin{aligned} l^A(m_0) &= 2 \sum_{i=1}^{n+1} \left( \lambda^A W_{ni}(m_0) - \frac{1}{2} (\lambda^A)^2 W_{ni}(m_0)^2 \right) + o_p(1) \\ &= \frac{n \overline{W}_n^2(m_0)}{S_n^2(m_0)} + o_p(1). \end{aligned}$$

Thus,  $l^A(m_0)$  converges to  $\chi_1^2$  by using Lemmas B.1 and B.2.

□

## Appendix C

### PROOFS OF CHAPTER 4

The following lemmas are needed for the proof of the theorems.

**Lemma C.1.** *Let  $V_i$ ,  $W_j$ ,  $V_{mi}$  and  $W_{nj}$  be defined by equations (4.3), (4.4), (4.5) and (4.6), respectively. Under the assumptions of Theorem 4.2.1, as  $m \rightarrow \infty$ ,  $n \rightarrow \infty$ , we have*

$$(i) \quad \frac{1}{m} \sum_{i=1}^m (V_{mi} - V_i)^2 = o_p(1),$$

$$(ii) \quad \max_{1 \leq i \leq m} |V_{mi} - \theta_1| = o_p(m^{1/2}), \quad \frac{1}{m} \sum_{i=1}^m (V_{mi} - \theta_1)^2 = \sigma_1^2 + o_p(1),$$

$$(iii) \quad \frac{1}{\sqrt{m}} \sum_{i=1}^m (V_{mi} - \theta_1) \rightarrow N(0, \sigma_1^2) \text{ in distribution,}$$

where  $\sigma_1^2 = \text{Var}(V_i)$ ,

$$(iv) \quad \frac{1}{n} \sum_{j=1}^n (W_{nj} - W_j)^2 = o_p(1),$$

$$(v) \quad \max_{1 \leq j \leq n} |W_{nj} - \theta_2| = o_p(n^{1/2}), \quad \frac{1}{n} \sum_{j=1}^n (W_{nj} - \theta_2)^2 = \sigma_2^2 + o_p(1),$$

$$(vi) \quad \frac{1}{\sqrt{n}} \sum_{j=1}^n (W_{nj} - \theta_2) \rightarrow N(0, \sigma_2^2) \text{ in distribution,}$$

where  $\sigma_2^2 = \text{Var}(W_j)$ .

*Proof.* See the proof of Lemma 3.1 in the Appendix of He et al. (2016) for (i), (ii), (iv) and (v).

Noting that  $\int_0^\infty (s - \theta_1) dF(s) = 0$ , we can write

$$\begin{aligned} \frac{1}{\sqrt{m}} \sum_{i=1}^m (V_{mi} - \theta_1) &= \sqrt{m} \int_0^\infty (s - \theta_1) d\widehat{F}_m(s) \\ &= \sqrt{m} \int_0^\infty (s - \theta_1) d(\widehat{F}_m(s) - F(s)). \end{aligned}$$

We obtain the relation (iii) from Corollary 1.2 in Stute (1995). We can prove (vi) similarly.  $\square$

**Lemma C.2.** *Let  $Z^V$  and  $Z^W$  be defined by equations (4.14) and (4.15). Under the assumptions of Theorem 4.2.1, as  $s \rightarrow \infty$ , we have*

$$(i) \quad \max_{1 \leq r \leq M} |Z_r^V - \theta_1| = o_p(s^{1/2}), \quad \max_{1 \leq s \leq N} |Z_s^W - \theta_1 + \theta| = o_p(s^{1/2})$$

$$(ii) \quad \frac{1}{M} \sum_{r=1}^M (Z_r^V - \theta_1)^2 = \frac{\sigma_1^2}{2} + o_p(1), \quad \frac{1}{N} \sum_{s=1}^N (Z_s^W - \theta_1 + \theta)^2 = \frac{\sigma_2^2}{2} + o_p(1).$$

*Proof.* Recall  $s = m + n$ . Let us note that under the condition  $m/s = \delta \rightarrow \delta_0 \in (0, 1)$  as  $s \rightarrow \infty$ ,  $O_p(m^{-1/2})$ ,  $O_p(n^{-1/2})$  and  $O_p(s^{-1/2})$  are all equivalent.

(i) We can write

$$\begin{aligned} \max_{1 \leq r \leq M} |Z_r^V - \theta_1| &= \max_{1 \leq k \leq l \leq m} \left| \frac{V_{mk} - \theta_1 + V_{ml} - \theta_1}{2} \right| \\ &\leq \frac{1}{2} \left( \max_{1 \leq k \leq m} |V_{mk} - \theta_1| + \max_{l \leq l \leq m} |V_{ml} - \theta_1| \right) \\ &= \frac{1}{2} (o_p(m^{1/2}) + o_p(m^{1/2})) \\ &= o_p(m^{1/2}) \\ &= o_p(s^{1/2}). \end{aligned}$$

Similarly,  $\max_{1 \leq s \leq N} |Z_s^W - \theta_1 + \theta| = o_p(s^{1/2})$ .

(ii) We also have

$$\begin{aligned} \frac{1}{M} \sum_{r=1}^M (Z_r^V - \theta_1)^2 &= \frac{1}{2M} \left( \sum_{k=1}^m \sum_{l=1}^m \left( \frac{V_{mk} - \theta_1 + V_{ml} - \theta_1}{2} \right)^2 + \sum_{k=1}^m (V_{mk} - \theta_1)^2 \right) \\ &= \frac{1}{2} \times \frac{1}{m} \sum_{k=1}^m (V_{mk} - \theta_1)^2 + \frac{1}{2(m+1)} \left( \frac{1}{\sqrt{m}} \sum_{k=1}^m (V_{mk} - \theta_1) \right)^2 \\ &= \frac{\sigma_1^2}{2} + o_p(1). \end{aligned}$$

In the same way,  $\frac{1}{N} \sum_{s=1}^N (Z_s^W - \theta_1 + \theta)^2 = \sigma_2^2/2 + o_p(1)$ .  $\square$

*Proof of Theorem 4.2.2.* Firstly, to maximize  $\log R(\theta_1, \theta)$ , we set the derivative  $\partial \log R(\theta_1, \theta) / \partial \theta_1 = 0$ , which leads to

$$m\lambda_1 + n\lambda_2 = 0. \quad (\text{C.1})$$

Secondly, following the same argument in the proof of Theorem 2.2 of Owen (2001), we can write that

$$\begin{aligned} \lambda_1 &= \frac{\frac{1}{m} \sum_{i=1}^m (V_{mi} - \theta_1)}{\frac{1}{m} \sum_{i=1}^m (V_{mi} - \theta_1)^2} + o_p(m^{-1/2}) \\ &= O_p(m^{-1/2}). \end{aligned} \quad (\text{C.2})$$

This relationship coupled with the second relationship in (ii) from Lemma C.1 gives

$$\begin{aligned} \lambda_1 &= \frac{\bar{V}_m - \theta_1}{\sigma_1^2 + o_p(1)} + o_p(m^{-1/2}) \\ &= O_p(m^{-1/2}) \\ &= O_p(s^{-1/2}), \end{aligned} \quad (\text{C.3})$$

and similarly

$$\begin{aligned} \lambda_2 &= \frac{\bar{W}_n - \theta_1 + \theta}{\sigma_2^2 + o_p(1)} + o_p(n^{-1/2}) \\ &= O_p(n^{-1/2}) \\ &= O_p(s^{-1/2}), \end{aligned} \quad (\text{C.4})$$

with  $\bar{V}_m = 1/m \sum_{i=1}^m V_{mi}$  and  $\bar{W}_n = 1/n \sum_{j=1}^n W_{nj}$ .

Replacing  $\lambda_1$  and  $\lambda_2$  in equation (C.1), we obtain

$$\hat{\theta}_1 = \frac{\frac{m}{\sigma_1^2}}{\frac{m}{\sigma_1^2} + \frac{n}{\sigma_2^2}} \bar{V}_m + \frac{\frac{n}{\sigma_1^2}}{\frac{m}{\sigma_1^2} + \frac{n}{\sigma_2^2}} (\bar{W}_n + \theta) + o_p(s^{-1/2}).$$

We note that

$$\begin{aligned}
\max_{1 \leq i \leq m} |V_{mi} - \hat{\theta}_1| &= \max_{1 \leq i \leq m} |V_{mi} - \theta_1 + \theta_1 - \hat{\theta}_1| \\
&\leq \max_{1 \leq i \leq m} |V_{mi} - \theta_1| + |\hat{\theta}_1 - \theta_1| \\
&= o_p(s^{1/2}) + o_p(1) \\
&= o_p(s^{1/2}),
\end{aligned}$$

and

$$\begin{aligned}
\sum_{i=1}^m |V_{mi} - \hat{\theta}_1|^2 &= \sum_{i=1}^m |V_{mi} - \theta_1 + \theta_1 - \hat{\theta}_1|^2 \\
&\leq \sum_{i=1}^m |V_{mi} - \theta_1|^2 + 2|\theta_1 - \hat{\theta}_1| \sum_{i=1}^m |V_{mi} - \theta_1| + \sum_{i=1}^m |\theta_1 - \hat{\theta}_1|^2 \\
&= O_p(s) + O_p(s^{-1/2}) O_p(s^{1/2}) + O_p(1) \\
&= O_p(s).
\end{aligned}$$

**Remark C.0.1.** In fact when  $\theta_1$  is replaced by  $\hat{\theta}_1$ , all the relationships or equations involving  $\hat{\theta}_1$  are preserved due to the asymptotic normality (equation (4.10)) and the consistency of  $\hat{\theta}_1$ . Either  $|\theta_1 - \hat{\theta}_1| = O_p(s^{-1/2})$  or  $|\theta_1 - \hat{\theta}_1| = o_p(1)$ .

Finally, applying the Taylor expansion to  $\log R(\theta, \theta_1)$  at  $\theta_1 = \hat{\theta}_1$  and  $\theta = \theta_0$ , we obtain the following expansion for  $\log r(\theta_0)$

$$\begin{aligned}
-2 \log r(\theta_0) &= -2 \log R(\hat{\theta}_1, \theta_0) \\
&= 2 \sum_{i=1}^m \left\{ \lambda_1(V_{mi} - \hat{\theta}_1) - \frac{1}{2} \left\{ \lambda_1(V_{mi} - \hat{\theta}_1) \right\}^2 \right\} + r_m \\
&+ 2 \sum_{j=1}^n \left\{ \lambda_2(W_{mj} - \hat{\theta}_1 + \theta_0) - \frac{1}{2} \left\{ \lambda_2(W_{mj} - \hat{\theta}_1 + \theta_0) \right\}^2 \right\} + r_n,
\end{aligned} \tag{C.5}$$

with

$$\begin{aligned}
|r_m| &\leq C_m \sum_{i=1}^m \left\{ \lambda_1 \left( V_{mi} - \widehat{\theta}_1 \right) \right\}^3 \\
&\leq C_m |\lambda_1|^3 \max_{1 \leq i \leq m} |V_{mi} - \widehat{\theta}_1| \sum_{i=1}^m |V_{mi} - \widehat{\theta}_1|^2 \\
&= O_p(s^{-3/2}) o_p(s^{1/2}) O_p(s) \\
&= o_p(1),
\end{aligned}$$

for some finite  $C_m \geq 0$ . Similarly  $|r_n| = o_p(1)$ . Let  $\bar{V} = 1/m \sum_{i=1}^m V_i$  and  $\bar{W} = 1/n \sum_{j=1}^n W_j$ .

Noting that

$$\begin{aligned}
\lambda_1 \left( V_{mi} - \widehat{\theta}_1 \right) &= \lambda_1 (V_{mi} - \theta_1) + \lambda_1 (\theta_1 - \widehat{\theta}_1) \\
&= o_p(1) + o_p(1) \\
&= o_p(1),
\end{aligned}$$

and  $\lambda_2 \left( W_{nj} - \widehat{\theta}_1 + \theta_0 \right) = o_p(1)$  and substituting  $\lambda_1$  and  $\lambda_2$  in  $\log r(\theta_0)$ , we have

$$\begin{aligned}
-2 \log r(\theta_0) &= \frac{m}{\sigma_1^2} \left( \bar{V}_m - \widehat{\theta}_1 \right)^2 + \frac{n}{\sigma_2^2} \left( \bar{W}_n - \widehat{\theta}_1 + \theta_0 \right)^2 + o_p(1) \\
&= \left( \bar{V}_m - \bar{W}_n - \theta_0 \right)^2 / \left( \frac{\sigma_1^2}{m} + \frac{\sigma_2^2}{n} \right) + o_p(1) \\
&= \left( \bar{V} - \bar{W} - \theta_0 \right)^2 / \left( \frac{\sigma_1^2}{m} + \frac{\sigma_2^2}{n} \right) + o_p(1) \\
&\xrightarrow{D} \chi_1^2,
\end{aligned}$$

as  $s \rightarrow \infty$ . □

*Proof of Theorem 4.2.3.* Recall  $t = m + n + 2$  and  $(m + 1)/t \rightarrow \rho \in (0, 1)$ . this implies that as  $t \rightarrow \infty$ ,  $O_p(m^{-1/2})$ ,  $O_p(n^{-1/2})$  and  $O_p(t^{-1/2})$  are all equivalent.

We first, show that  $|\lambda_1^A| = O_p(t^{-1/2})$  and  $|\lambda_2^A| = O_p(t^{-1/2})$ .

From equation (4.12), we can write

$$\begin{aligned}
0 &= \frac{1}{m} \left| \sum_{i=1}^{m+1} (V_{mi} - \theta_1) - \lambda_1^A \sum_{i=1}^{m+1} \frac{(V_{mi} - \theta_1)^2}{1 + \lambda_1^A (V_{mi} - \theta_1)} \right| \\
&\geq \frac{|\lambda_1^A|}{m} \sum_{i=1}^{m+1} \frac{(V_{mi} - \theta_1)^2}{1 + \lambda_1^A (V_{mi} - \theta_1)} - \left| \frac{1}{m} \sum_{i=1}^{m+1} (V_{mi} - \theta_1) \right| \\
&\geq \frac{|\lambda_1^A| s_m^2}{1 + |\lambda_1^A| V_m^*} - \left| \frac{1}{m} \sum_{i=1}^m (V_{mi} - \theta_1) \right| \left( 1 - \frac{a_m}{m} \right),
\end{aligned}$$

where  $s_m^2 = m^{-1} \sum_{i=1}^m (V_{mi} - \theta_1)^2$  and  $V_m^* = \max_{1 \leq i \leq m} |V_{mi} - \theta_1|$ . By Lemma D.1, one has  $m^{-1} \sum_{i=1}^m [V_{mi} - \theta_1] = O_p(m^{-1/2})$ ,  $s_m^2 = \sigma_1^2 + o_p(1)$  and  $V_m^* = o_p(m^{1/2})$ . With  $a_m = o_p(m)$ , we get  $|\lambda_1^A|/(1 + |\lambda_1^A| o_p(m^{1/2})) = O_p(m^{-1/2})$  and therefore  $|\lambda_1^A| = O_p(m^{-1/2}) = O_p(t^{-1/2})$ . Similarly, we can show that  $|\lambda_2^A| = O_p(t^{-1/2})$ .

Next, we have

$$\begin{aligned}
0 &= \frac{1}{m} \sum_{i=1}^{m+1} \frac{V_{mi} - \theta_1}{1 + \lambda_1^A (V_{mi} - \theta_1)} \\
&= \frac{1}{m} \sum_{i=1}^{m+1} (V_{mi} - \theta_1) - \frac{1}{m} \sum_{i=1}^{m+1} \frac{\lambda_1^A (V_{mi} - \theta_1)^2}{1 + \lambda_1^A (V_{mi} - \theta_1)} \\
&= \bar{V}_m - \theta_1 - \lambda_1^A s_m^2 + o_p(m^{-1/2}).
\end{aligned}$$

As  $t \rightarrow \infty$ , we have  $\lambda_1^A = (\bar{V}_m - \theta_1)/s_m^2 + o_p(t^{-1/2})$ . Similarly,  $\lambda_2^A = (\bar{W}_n - \theta_1 + \theta)/s_n^2 + o_p(t^{-1/2})$ . We then maximize  $\log R^A(\theta_1, \theta)$ , by setting the derivative  $\partial \log R^A(\theta_1, \theta) / \partial \theta_1 = 0$ , and obtain

$$(m+1)\lambda_1^A + (n+1)\lambda_2^A = 0. \quad (\text{C.6})$$

We can find the value of  $\hat{\theta}_1^A$ , which maximizes  $\theta_1$  by replacing  $\lambda_1^A$ ,  $\lambda_2^A$ , and  $s_m^2$  by their respective values in equation (C.6).

The rest of the proof is similar to the proof of Theorem 4.2.2. We have

$$\begin{aligned}
-2 \log r^A(\theta_0) &= -2 \log R^A(\hat{\theta}_1^A, \theta_0) \\
&\xrightarrow{\mathcal{D}} \chi_1^2,
\end{aligned}$$

as  $t \rightarrow \infty$ . □

*Proof of Theorem 4.2.4.* We first prove that  $|\lambda_1^M| = O_p(s^{-1/2})$  and  $|\lambda_2^M| = O_p(s^{-1/2})$ . From Lemma D.2, we obtain  $\frac{1}{M} \sum_{r=1}^M (Z_r^V - \theta_1)^2 = \frac{\sigma_1^2}{2} + o_p(1) = O_p(1)$ . Noticing that

$$\begin{aligned} \frac{1}{M} \sum_{r=1}^M (Z_r^V - \theta_1) &= \frac{1}{2M} \left( \sum_{k=1}^m \sum_{l=1}^m \left( \frac{V_{mk} - \theta_1 + V_{ml} - \theta_1}{2} \right) + \sum_{k=1}^m (V_{mk} - \theta_1) \right) \\ &= \frac{m+1}{2M} \sum_{k=1}^m (V_{mk} - \theta_1) \\ &= \bar{V}_m - \theta_1 \\ &= \bar{V} - \theta_1 \\ &= O_p(m^{-1/2}) \\ &= O_p(s^{-1/2}), \end{aligned}$$

and following the same arguments as in Owen(1990), we have  $|\lambda_1^M| = O_p(s^{-1/2})$ .  $|\lambda_2^M| = O_p(s^{-1/2})$  can be proved similarly. Now expanding the equation (4.17), we have

$$\begin{aligned} 0 &= \frac{1}{M} \sum_{r=1}^M \frac{Z_r^V - \theta_1}{1 + \lambda_1^M (Z_r^V - \theta_1)} \\ &= \frac{1}{M} \sum_{r=1}^M (Z_r^V - \theta_1) - \frac{\lambda_1^M}{M} \sum_{r=1}^M (Z_r^V - \theta_1)^2 + \frac{1}{M} \sum_{r=1}^M \frac{(\lambda_1^M)^2 (Z_r^V - \theta_1)^3}{1 + \lambda_1^M (Z_r^V - \theta_1)} \\ &= \bar{V} - \theta_1 - \lambda_1^M \frac{\sigma_1^2}{2} + \gamma_s, \end{aligned}$$

where

$$\begin{aligned} \gamma_s &\leq (\lambda_1^M)^2 \max_{1 \leq r \leq M} |Z_r^V - \theta_1| \frac{1}{M} \sum_{r=1}^M (Z_r^V - \theta_1)^2 \times \frac{1}{1 + \lambda_1^M (Z_r^V - \theta_1)} \\ &= O_p(s^{-1}) o_p(s^{1/2}) O_p(1) O_p(1) \\ &= o_p(s^{-1/2}). \end{aligned}$$

We have then  $\lambda_1^M = 2(\bar{V} - \theta_1)/\sigma_1^2 + o_p(s^{-1/2})$  and  $\lambda_2^M = 2(\bar{W} - \theta_1 + \theta)/\sigma_2^2 + o_p(s^{-1/2})$  as



well. Once again we set the derivative  $\partial \log R^{\mathcal{M}}(\theta_1, \theta) / \partial \theta_1 = 0$ , which leads to

$$M\lambda_1^{\mathcal{M}} + N\lambda_2^{\mathcal{M}} = 0. \quad (\text{C.7})$$

And by substituting  $\lambda_1^{\mathcal{M}}$  and  $\lambda_2^{\mathcal{M}}$  by their expressions in equation (C.7), we can find  $\widehat{\theta}_1^{\mathcal{M}}$ , which maximizes  $\log R^{\mathcal{M}}(\theta_1, \theta)$ . As in the previous proofs, applying the Taylor expansion to  $\log R^{\mathcal{M}}(\theta_1, \theta)$  at  $\theta_1 = \widehat{\theta}_1^{\mathcal{M}}$  and  $\theta = \theta_0$ , we obtain the following expansion for  $\log r(\theta_0)$

$$\begin{aligned} \frac{-2 \log r^{\mathcal{M}}(\theta_0)}{s} &= -\frac{2}{s} \log R^{\mathcal{M}}(\widehat{\theta}_1^{\mathcal{M}}, \theta_0) \\ &= \frac{2}{s} \sum_{r=1}^M \left\{ \lambda_1^{\mathcal{M}} (Z_r^V - \widehat{\theta}_1^{\mathcal{M}}) - \frac{1}{2} \left\{ \lambda_1^{\mathcal{M}} (Z_r^V - \widehat{\theta}_1^{\mathcal{M}}) \right\}^2 \right\} \\ &+ \frac{2}{s} \sum_{s=1}^N \left\{ \lambda_2^{\mathcal{M}} (Z_s^W - \widehat{\theta}_1^{\mathcal{M}} + \theta_0) - \frac{1}{2} \left\{ \lambda_2^{\mathcal{M}} (Z_s^W - \widehat{\theta}_1^{\mathcal{M}} + \theta_0) \right\}^2 \right\} + \frac{r_S}{s}, \end{aligned} \quad (\text{C.8})$$

with

$$\begin{aligned} |r_S| &\leq C_M \sum_{r=1}^M \left\{ \lambda_1^{\mathcal{M}} (Z_r^V - \widehat{\theta}_1^{\mathcal{M}}) \right\}^3 + C_N \sum_{s=1}^N \left\{ \lambda_2^{\mathcal{M}} (Z_s^W - \widehat{\theta}_1^{\mathcal{M}} + \theta_0) \right\}^3 \\ &\leq C_M |\lambda_1^{\mathcal{M}}|^3 \max_{1 \leq r \leq M} |Z_r^V - \widehat{\theta}_1^{\mathcal{M}}| \sum_{r=1}^M |Z_r^V - \widehat{\theta}_1^{\mathcal{M}}|^2 \\ &+ C_N |\lambda_2^{\mathcal{M}}|^3 \max_{1 \leq s \leq N} |Z_s^W - \widehat{\theta}_1^{\mathcal{M}} + \theta_0| \sum_{s=1}^N |Z_s^W - \widehat{\theta}_1^{\mathcal{M}} + \theta_0|^2 \\ &= O_p(s^{-3/2}) o_p(s^{1/2}) O_p(s^2) + O_p(s^{-3/2}) o_p(s^{1/2}) O_p(s^2) \\ &= o_p(s) + o_p(s) \\ &= o_p(s), \end{aligned}$$

for some finite  $C_M \geq 0$  and  $C_N \geq 0$ .

By plugging in  $\lambda_1^{\mathcal{M}}$  and  $\lambda_2^{\mathcal{M}}$  in equation (C.8) and after some algebra, we have

$$\begin{aligned}
\frac{-2 \log r^{\mathcal{M}}(\theta_0)}{s} &= \frac{2M}{s\sigma_1^2}(\bar{V}_m - \hat{\theta}_1^{\mathcal{M}})^2 + \frac{2N}{s\sigma_2^2}(\bar{W}_n - \hat{\theta}_1^{\mathcal{M}} + \theta_0)^2 + o_p(1) \\
&= s(\bar{V}_m - \bar{W}_n - \theta_0)^2 / \left( \frac{s^2\sigma_1^2}{2M} + \frac{s^2\sigma_2^2}{2N} \right) + o_p(1) \\
&= s(\bar{V}_m - \bar{W}_n - \theta_0)^2 / \left( \frac{\sigma_1^2}{\delta^2} + \frac{\sigma_2^2}{(1-\delta)^2} \right) + o_p(1) \\
&= s(\bar{V} - \bar{W} - \theta_0)^2 / \left( \frac{\sigma_1^2}{\delta^2} + \frac{\sigma_2^2}{(1-\delta)^2} \right) + o_p(1).
\end{aligned} \tag{C.9}$$

Applying the Central Limit Theorem, we can write

$$\begin{aligned}
(\bar{V} - \bar{W} - \theta_0)^2 / \left( \frac{\sigma_1^2}{m} + \frac{\sigma_2^2}{n} \right) + o_p(1) &= s(\bar{V} - \bar{W} - \theta_0)^2 / \left( \frac{\sigma_1^2}{\delta} + \frac{\sigma_2^2}{1-\delta} \right) + o_p(1) \\
&\xrightarrow{\mathcal{D}} \chi_1^2.
\end{aligned} \tag{C.10}$$

Combining equations (D.9) and (D.10), with  $\delta \rightarrow \delta_0$ , we have

$$-\frac{2}{s} \log r^{\mathcal{M}}(\theta_0) \xrightarrow{\mathcal{D}} r\chi_1^2,$$

as  $s \rightarrow \infty$ ,

$$\text{where } r = \left( \frac{\sigma_1^2}{\delta_0} + \frac{\sigma_2^2}{1-\delta_0} \right) / \left( \frac{\sigma_1^2}{\delta_0^2} + \frac{\sigma_2^2}{(1-\delta_0)^2} \right).$$

□

## Appendix D

### PROOFS OF CHAPTER 5

Let us assume the following regularity conditions:

- 1)  $F, G, K,$  and  $Q$  are continuous,
- 2)  $m/n \rightarrow \rho$  as  $m, n \rightarrow \infty$  with  $0 < \rho < \infty$ ,
- 3)  $\sqrt{m+n} \int_{X_{(m)}}^{\tau_F} F_k(t) dG_k(t) \xrightarrow{P} 0$ ,
- 4)  $\sqrt{m+n} (G_k(\tau_F) - G_k(X_{(m)})) \xrightarrow{P} 0$ ,
- 5)  $\sup_t \left| \int_t^{\tau_F} (1 - F_k(s)) dG_k(s) / (1 - F_k(t)) \right|$ ,
- 6)  $\int_0^{\tau_F} dF_k(s) / (1 - K_k(s-)) < \infty$ ,
- 7)  $\int_0^{\tau_F} dG_k(s) / (1 - Q_k(s-)) < \infty$ ,
- 8)  $N_{x_{ki}} = I(X_{ki} \leq t, \delta_{ki} = 1)$  and  $N_{y_{kj}} = I(Y_{kj} \leq t, \eta_{kj} = 1)$  have no common jumps.

These assumptions are borrowed from Wang et al. (2009) and assure the existence of their proposed estimator for the AUC under right censoring. The following lemma is needed for the proofs of the theorems.

**Lemma D.1.** *Assume that the regularity conditions hold. Then, as  $n \rightarrow \infty$ , we have*

$$\frac{1}{\sum \omega_{kl}} \sum_{j=1}^n \omega_{kj} \left( \widehat{Z}_{kj} - \Delta_k^0 \right)^2 \xrightarrow{P} \sigma_k^{0^2},$$

where  $\sigma_k^{0^2} = \text{Var}(Z_{k1})$ .

*Proof.*

$$\begin{aligned} S_k^2 &= \frac{1}{\sum \omega_{kl}} \sum_{j=1}^n \omega_{kj} \left( \widehat{Z}_{kj} - \Delta_k^0 \right)^2 = \int_0^\infty \widehat{F}_k(t) d\widehat{G}_k(t) + \Delta_k^0 \left( \Delta_k^0 - 2\widehat{\Delta}_k \right) \\ &\xrightarrow{P} \int_0^\infty F_k^2(t) dG_k(t) - \Delta_k^{0^2} \end{aligned}$$

for  $k = 1, 2$  as  $\widehat{\Delta}_k$  is a consistent estimator of  $\Delta_k$ .

$$\int_0^\infty F_k^2(t) dG_k(t) - \Delta_k^{0^2} = E_{G_k}(Z_{k1} - \Delta_k^0)^2 = \sigma_k^{0^2}. \quad \square$$

*Proof of Theorem 5.2.2.* We can write

$$\begin{aligned} \sqrt{m+n} \left( \widehat{\Delta} - \Delta^0 \right) &= \sqrt{m+n} \left( \widehat{\Delta}_1 - \Delta_1^0 \right) - \sqrt{m+n} \left( \widehat{\Delta}_2 - \Delta_2^0 \right) \\ &= \sqrt{1 + \frac{n}{m}} (\alpha_{11} - \alpha_{12}) + \sqrt{1 + \frac{m}{n}} (\beta_{11} - \beta_{12}), \end{aligned} \quad (\text{D.1})$$

where  $\alpha_{1k}$  and  $\beta_{1k}$  are defined by equations (5.5) and (5.6), respectively. Note that  $\alpha_{1k}$  and  $\beta_{1k}$  correspond to  $\alpha_{n,m,1}$  and  $\beta_{n,m,1}$  in the Appendix of Wang et al. (2009). Equation (D.1) is then the martingale representation of  $\sqrt{m+n} \left( \widehat{\Delta} - \Delta^0 \right)$ .

Noting that  $\text{Cov}(\alpha_{1k}, \beta_{1k}) = 0, k = 1, 2$  by the independence of  $X_k$  and  $Y_k$ , we have

$$\begin{aligned} \text{Var} \left( \sqrt{m+n} \left( \widehat{\Delta} - \Delta^0 \right) \right) &= \left( 1 + \frac{n}{m} \right) \text{Var}(\alpha_{11} - \alpha_{12}) + \left( 1 + \frac{m}{n} \right) \text{Var}(\beta_{11} - \beta_{12}) \\ &\quad - 2\sqrt{\left( 1 + \frac{n}{m} \right) \left( 1 + \frac{m}{n} \right)} \text{Cov}(\alpha_{11} - \alpha_{12}, \beta_{11} - \beta_{12}) \\ &= \left( 1 + \frac{n}{m} \right) \text{Var}(\alpha_{11} - \alpha_{12}) + \left( 1 + \frac{m}{n} \right) \text{Var}(\beta_{11} - \beta_{12}) \\ &= \left( 1 + \frac{n}{m} \right) (\text{Var}(\alpha_{11}) + \text{Var}(\alpha_{12}) - 2\text{Cov}(\alpha_{11}, \alpha_{12})) \\ &\quad + \left( 1 + \frac{m}{n} \right) (\text{Var}(\beta_{11}) + \text{Var}(\beta_{12}) - 2\text{Cov}(\beta_{11}, \beta_{12})) \\ &= \left( 1 + \frac{1}{\rho} \right) (\sigma_{x1}^2 + \sigma_{x2}^2) + (1 + \rho) (\sigma_{y1}^2 + \sigma_{y2}^2) \\ &\quad - 2 \left( 1 + \frac{1}{\rho} \right) \text{Cov}(\alpha_{11}, \alpha_{12}) - 2(1 + \rho) \text{Cov}(\beta_{11}, \beta_{12}) \\ &= \sigma_1^2 + \sigma_2^2 - 2\sigma_{12}^2. \end{aligned}$$

$\square$

*Proof of Theorem 5.2.3.* Next, using Lemma D.1 and similar methods used in Owen (1990),

we can prove  $|\lambda| = O_p(n^{-1/2})$ . Let  $V_k = \max_j |\widehat{Z}_{kj} - \Delta_k^0|$ ,  $k = 1, 2$ . From equation (5.7) we can write

$$\begin{aligned}
0 &= \frac{1}{\sum \omega_{1l}} \left| \sum_{j=1}^n \frac{\omega_{1j} (\widehat{Z}_{1j} - \Delta_1^0)}{1 + 2\lambda (\widehat{Z}_{1j} - \Delta_1^0)} \right| \\
&= \frac{1}{\sum \omega_{1l}} \left| \sum_{j=1}^n (\widehat{Z}_{1j} - \Delta_1^0) - 2\lambda \sum_{j=1}^n \frac{\omega_{1j} (\widehat{Z}_{1j} - \Delta_1^0)^2}{1 + 2\lambda (\widehat{Z}_{1j} - \Delta_1^0)} \right| \\
&\geq \left| \frac{2\lambda}{\sum \omega_{1l}} \sum_{j=1}^n \frac{\omega_{1j} (\widehat{Z}_{1j} - \Delta_1^0)^2}{1 + 2\lambda (\widehat{Z}_{1j} - \Delta_1^0)} \right| - \frac{1}{\sum \omega_{1l}} \left| \sum_{j=1}^n (\widehat{Z}_{1j} - \Delta_1^0) \right| \\
&\geq \frac{|2\lambda| S_1^2}{1 + |2\lambda| V_1} - \frac{1}{\sum \omega_{1l}} \left| \sum_{j=1}^n (\widehat{Z}_{1j} - \Delta_1^0) \right| \\
&\geq \frac{S_1^2}{1 + |2\lambda| V_1} - \frac{1}{\sum \omega_{1l}} \frac{\left| \sum_{j=1}^n (\widehat{Z}_{1j} - \Delta_1^0) \right|}{|2\lambda|}.
\end{aligned}$$

It is clear that  $V_1 = O_p(1)$ . By Lemma D.1,  $S_1^2 = O_p(1)$ , and by Theorem 5.2.1

$$\begin{aligned}
\frac{1}{\sum \omega_{kl}} \sum_{j=1}^n \omega_{kj} (\widehat{Z}_{kj} - \Delta_k) &= \widehat{\Delta}_k - \Delta_k^0 \\
&= O_p((m+n)^{-1/2}) \\
&= O_p(n^{-1/2}), \quad k = 1, 2.
\end{aligned}$$

Therefore  $|2\lambda| = O_p(n^{-1/2})$  and  $|\lambda| = O_p(n^{-1/2})$ .

Since  $\max_{1 \leq i \leq n} |\widehat{Z}_{kj} - \Delta_k^0| = O_p(1)$ , applying Taylor's expansion, we can write

$$\begin{aligned}
\frac{1}{2} l(\Delta^0) &= \sum_{j=1}^n \omega_{1j} \left\{ 2\lambda (\widehat{Z}_{1j} - \Delta_1^0) - \frac{1}{2} (2\lambda (\widehat{Z}_{1j} - \Delta_1^0))^2 \right\} + R_{1n} \\
&+ \sum_{j=1}^n \omega_{2j} \left\{ -2\lambda (\widehat{Z}_{2j} - \Delta_2^0) + \frac{1}{2} (2\lambda (\widehat{Z}_{2j} - \Delta_2^0))^2 \right\} + R_{2n},
\end{aligned} \tag{D.2}$$

where

$$R_{kn} \leq C \sum_{j=1}^n \omega_{kj} |\lambda (\widehat{Z}_{kj} - \Delta_k^0)|^3 \leq C |\lambda|^3 n = O_p(n^{-1/2}), k = 1, 2.$$

Recall that  $\lambda$ ,  $\Delta_1^0$  and  $\Delta_2^0$  are solutions of

$$\frac{1}{\sum \omega_{1l}} \sum_{j=1}^n \frac{\omega_{1j} (\widehat{Z}_{1j} - \Delta_1^0)}{1 + 2\lambda (\widehat{Z}_{1j} - \Delta_1^0)} = 0, \quad (\text{D.3})$$

$$\frac{1}{\sum \omega_{2l}} \sum_{j=1}^n \frac{\omega_{2j} (\widehat{Z}_{2j} - \Delta_2^0)}{1 - 2\lambda (\widehat{Z}_{2j} - \Delta_2^0)} = 0, \quad (\text{D.4})$$

$$\frac{1}{\sum \omega_{1l}} \sum_{j=1}^n \frac{\omega_{1j} \widehat{Z}_{1j}}{1 + 2\lambda (\widehat{Z}_{1j} - \Delta_1^0)} - \frac{1}{\sum \omega_{2l}} \sum_{j=1}^n \frac{\omega_{2j} \widehat{Z}_{2j}}{1 - 2\lambda (\widehat{Z}_{2j} - \Delta_2^0)} = \Delta^0. \quad (\text{D.5})$$

From equations (D.3) and (D.4), we have

$$2\lambda = \frac{\frac{1}{\sum \omega_{2l}} \sum_{j=1}^n \omega_{kj} (\widehat{Z}_{kj} - \Delta_k^0)}{\frac{1}{\sum \omega_{2l}} \sum_{j=1}^n \omega_{kj} (\widehat{Z}_{kj} - \Delta_k^0)^2} + o_p(n^{-1/2}),$$

$$\sum_{j=1}^n \omega_{kj} \cdot 2\lambda (\widehat{Z}_{kj} - \Delta_k^0) = \sum_{j=1}^n \omega_{kj} (2\lambda (\widehat{Z}_{kj} - \Delta_k^0))^2 + o_p(1). \quad (\text{D.6})$$

Substituting equation (D.6) in equation (D.2), we obtain

$$\begin{aligned}
l(\Delta^0) &= \sum_{j=1}^n \omega_{1j} \cdot 2\lambda \left( \widehat{Z}_{1j} - \Delta_1^0 \right) - \sum_{j=1}^n \omega_{2j} \cdot 2\lambda \left( \widehat{Z}_{2j} - \Delta_2^0 \right) + o_p(1) \\
&= 2\lambda \left\{ \sum_{j=1}^n \omega_{1j} \widehat{Z}_{1j} - \sum_{j=1}^n \omega_{2j} \widehat{Z}_{2j} - \Delta_1^0 \sum_{j=1}^n \omega_{1j} + \Delta_2^0 \sum_{j=1}^n \omega_{2j} \right\} + o_p(1) \\
&= 2\lambda \left\{ \sum \omega_{1l} \widehat{\Delta}_1 - \sum \omega_{2l} \widehat{\Delta}_2 - \left( \Delta_1^0 \sum_{j=1}^n \omega_{1j} - \Delta_2^0 \sum_{j=1}^n \omega_{2j} \right) \right\} + o_p(1) \\
&= 2\lambda n \left\{ \widehat{\Delta}_1 - \widehat{\Delta}_2 - (\Delta_1^0 - \Delta_2^0) \right\} + o_p(1), \text{ since } \sum \omega_{kl} = n \\
&= 2\lambda n \left( \widehat{\Delta} - \widehat{\Delta}^0 \right) + o_p(1). \tag{D.7}
\end{aligned}$$

Recall that

$$\frac{1}{\sum \omega_{kl}} \sum_{j=1}^n \omega_{kj} \left( \widehat{Z}_{kj} - \Delta_k \right) = o_p(1), \quad k = 1, 2.$$

From equation (D.5), we have

$$\begin{aligned}
&\frac{1}{\sum \omega_{1l}} \sum_{j=1}^n \omega_{1j} \left( \widehat{Z}_{1j} - \Delta_1^0 \right) \left( 1 + 2\lambda \left( \widehat{Z}_{1j} - \Delta_1^0 \right) \right) - \\
&\quad \frac{1}{\sum \omega_{2l}} \sum_{j=1}^n \omega_{2j} \left( \widehat{Z}_{2j} - \Delta_2^0 \right) \left( 1 - 2\lambda \left( \widehat{Z}_{2j} - \Delta_2^0 \right) \right) = o_p(\lambda). \tag{D.8}
\end{aligned}$$

Hence, we have

$$\begin{aligned}
2\lambda &= \frac{\frac{1}{\sum \omega_{1l}} \sum_{j=1}^n \omega_{1j} \widehat{Z}_{1j} - \frac{1}{\sum \omega_{2l}} \sum_{j=1}^n \omega_{2j} \widehat{Z}_{2j} - (\Delta_1^0 - \Delta_2^0)}{\frac{1}{\sum \omega_{1l}} \sum_{j=1}^n \omega_{1j} \left( \widehat{Z}_{1j} - \Delta_1^0 \right)^2 + \frac{1}{\sum \omega_{2l}} \sum_{j=1}^n \omega_{2j} \left( \widehat{Z}_{2j} - \Delta_2^0 \right)^2} + o_p(\lambda), \\
2\lambda &= \frac{\widehat{\Delta} - \Delta^0}{S_1^2 + S_2^2} + o_p(n^{-1/2}). \tag{D.9}
\end{aligned}$$

Thus, using equations D.7, D.9, Lemma D.1 and Theorem 5.2.2, we obtain that

$$\begin{aligned}
 r(\Delta^0)l(\Delta^0) &= r(\Delta^0)n \cdot \frac{(\widehat{\Delta} - \Delta^0)^2}{S_1^2 + S_2^2} + o_p(1) \\
 &= r(\Delta^0) \cdot \frac{n\sigma^2}{(m+n)(S_1^2 + S_2^2)} \left( \frac{\sqrt{m+n}(\widehat{\Delta} - \Delta^0)}{\sigma} \right)^2 + o_p(1) \\
 &= \left( \sqrt{m+n} \frac{\widehat{\Delta} - \Delta^0}{\sigma} \right)^2 + o_p(1) \\
 &\xrightarrow{\mathcal{D}} \chi_1^2.
 \end{aligned}$$

□

*Proof of Theorem 5.2.4.* The proof of Theorem 5.2.4 is similar to these of Theorem 2.2.2 in Chapter 2 and Theorem 3.2.2 in Chapter 3.

□