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# ASYMPTOTIC PROPERTIES OF CENSORED LINEAR RANK TESTS

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A conjecture of Prentice is established which states that for censored linear rank test, exact scores based on conditional expectations can be replaced by approximate scores obtained by evaluating the score function at an estimate of the survival function. We show that under minimal conditions, asymptotically equivalent tests are obtained when either the Kaplan-Meier, Altshuler, or moment estimator of the survival function is used. Asymptotic normality is also established for a general random censorship model under the null hypothesis, and for contiguous alternatives. This is used to calculate efficacies, and when the censoring times are i.i.d., an expression for the asymptotic relative efficiency is given which is a natural generalization of the one for classical uncensored linear rank tests.

**1. Introduction.** Prentice (1978) has constructed a general class of linear rank tests for a regression model with censored data. His approach was to specify a score function for a classical linear rank test in the absence of censoring (Chernoff and Savage, 1958; Hájek and Šidák, 1967), to construct all possible rankings of the (unobserved) uncensored values which were consistent with observed censored sample, and then to assign to each observation the average of all possible scores it could have received in the absence of censoring, giving equal weighting to each possible uncensored ranking. Except for a few special scoring functions (notably logarithmic or power-law scores), these averages are unwieldy when there is appreciable censoring, and Prentice suggested that asymptotically equivalent tests might arise if the score function was evaluated at some suitable estimate of the survivor function. This is well known to be true for uncensored data, but requires further justification when censoring is present, because the estimate of the survivor function is more complicated.

In this work we show that Prentice's conjecture is true under minimal conditions and also we show that it remains true if Prentice's moment estimator of the survivor function is replaced by either the Kaplan-Meier estimator or Altshuler's estimator. Asymptotic normality of these tests is established under a random censorship model for the null hypothesis and contiguous alternatives by martingale methods. This is used to compute efficacies for a general random censorship model and asymptotic relative efficiencies for i.i.d. potential censoring times, where fully efficient linear rank tests exist. Asymptotic normality for general alternatives has been established in Cuzick (1982) by more complicated methods.

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**2. Approximate scores.** Let $\{T_i\}$ be a sequence of random variables. We observe the sequence of pairs $(X_i, \varepsilon_i)$ where $X_i \le T_i$ and $\varepsilon_i = 0$ if $T_i$ is uncensored (i.e. $X_i = T_i$) or $\varepsilon_i = 1$ if $T_i$ is censored (i.e. $X_i < T_i$). To each observation we attach a covariate $z_i$. The 2-sample problem is obtained by letting $z_i$ denote group membership. Prentice's generalized rank vector for the $X_i$, $i = 1, \cdots, n$ can be represented as follows:

$$\mathbf{R}_n = (R_{1n}, \cdots, R_{nn}) \quad \text{and for} \quad i = 1, \cdots, n \quad R_{in} = (j, l)$$

where $j$ is the number of uncensored observations less than or equal to $X_i$ and $l$ is an indicator function for a censored observation, i.e. $l = \varepsilon_i$. Given a score function $\phi$, the score assigned to an observation with generalized rank $R_{in} = (j, l)$ is

$$J((j, l); \phi_l) = \int_{0 \le u_1 \cdots < u_k \le 1} \phi_l(u_j)\, d(u_1, \cdots, u_k), \quad l = 0, 1, \quad j = 1, \cdots, k$$

where $\phi_0 = \phi$, $\phi_1(u) = u^{-1} \int_0^u \phi_0(v)\, dv$, $k$ is the total number of uncensored observations,

$$d(u_1, \cdots, u_k) = \prod_{i=1}^{k} n_i u_i^{m_i}\, du_i,$$

$m_i$ is the number of censored observations between the $i$th and $(i + 1)$st uncensored observation, and $n_i = \sum_{l=i}^{k} (m_l + 1)$ is the number of observations greater than or equal to the $i$th largest uncensored value. By convention $J((0, 1); \chi) = \int_0^1 \chi(u)\, du$. We note in passing that under a progressive type II censoring model $J(R_{in}; \phi) = E(\phi \mid R_{in})$. In particular this holds when there is no censoring. (See Kalbfleisch and Prentice, 1980, for a discussion of censoring mechanisms).

Prentice's test statistic takes the form

$$\mathscr{T}^* = (1/n) \sum_{i=1}^{n} z_i J(R_{in}; \phi_{l_i}), \tag{1}$$

and he conjectured that an asymptotically equivalent statistic would arise if $J((j, l); \phi_l)$ were replaced by

$$\phi_l(J((j, l); u)) = \phi_l(\hat{F}_T^M),$$

where

$$\hat{F}_T^M = \prod_{i \le j} n_i/(n_i + 1)$$

which is the right continuous version of the moment estimator of the survival function when the $\{T_i\}$ are i.i.d.

This leads to the alternative statistic

$$\mathscr{T} = \frac{1}{n} \sum_{i=1}^{n} z_i \phi_{l_i}(\hat{F}_T^M) = \int \phi_0(\hat{F}_T^M)\, d\hat{F}_0^z + \int \phi_1(\hat{F}_T^M)\, d\hat{F}_1^z, \tag{2}$$

where

$$\hat{F}_0^z(t) = (1/n) \sum_{i=1}^{n} z_i(1 - \varepsilon_i) I_{\{X_i > t\}}$$

$$\hat{F}_1^z(t) = (1/n) \sum_{i=1}^{n} z_i \varepsilon_i I_{\{X_i > t\}},$$

and the integrals are over $(\infty, -\infty)$, i.e. the whole real line but with reversed polarity since $F_0^z$ and $F_1^z$ are decreasing. We also record here the notation

$$\hat{F}_0(t) = n^{-1} \sum_{i=1}^n (1 - \varepsilon_i) I_{\{X_i > t\}}, \quad \hat{F}_1(t) = n^{-1} \sum_{i=1}^n \varepsilon_i I_{\{X_i > t\}}$$

and $\hat{F} = \hat{F}_0 + \hat{F}_1$.

The correctness of this conjecture is confirmed by the following:

THEOREM 1. *Assume that the score function $\phi$ is twice continuously differentiable on* (0, 1) *and that*

$$|t\phi'(t)| + |t^2\phi''(t)| \le Kt^{-\alpha} \tag{3}$$

*for some $\alpha < 1/2$ and $K < \infty$.*

*Also assume that the $\{T_i\}$ have continuous distribution functions and that*

$$\liminf_{n\to\infty} n \operatorname{Var}(\mathscr{T}) > 0.$$

*Then the statistic* (1) *is asymptotically equivalent to* (2), *i.e. $J(R_{in}; \phi_l)$ may be replaced by $\phi_l(\hat{F}_T^M)$.*

An asymptotic approximation for $\operatorname{Var}(\mathscr{T})$ under a random censorship model with the $\{T_i\}$ i.i.d. is given in the next section, from which the growth condition may be checked. An expression for general alternatives is given in Cuzick (1982). We also note that Theorem 1 remains true if either the Kaplan-Meier (1958) estimator

$$\hat{F}_T^{KM} = \prod_{i \le j} ((n_i - 1)/n_i)$$

or Altshuler's (1970) estimator

$$\hat{F}_T = \exp(-\textstyle\sum_{i\le j} n_i^{-1})$$

is used in place of $\hat{F}_T^M$.

THEOREM 2. *The results of Theorem* 1 *remain valid if either $\hat{F}_T$ or $\hat{F}_T^{KM}$ is used in place of $\hat{F}_T^M$ in* (2).

Before proving these results we require the following:

LEMMA. *Let $n_1 > n_2 \cdots > n_k \ge 1$ be positive integers. Then for any $j \le k$*

$$\prod_{i=1}^j \frac{n_i - 1}{n_i} < \exp\left\{-\sum_{i=1}^j \frac{1}{n_i}\right\} < \prod_{i=1}^j \frac{n_i}{n_i + 1}$$

*and*

$$\left| \prod_{i=1}^j \frac{n_i}{n_i + 1} - \prod_{i=1}^j \frac{n_i - 1}{n_i} \right| < \left\{\prod_{i=1}^j \frac{n_i}{n_i + 1}\right\} \frac{2}{n_j}. \tag{4}$$

PROOF. A Taylor expansion of $e^{-x}$ gives

$$1 - 1/n < e^{-1/n} < 1 - 1/n + 1/2n^2$$

and $1 - 1/n + 1/2n^2 \le 1 - (n + 1)^{-1}$ for $n \ge 1$, from which the first pair of inequalities follow. To establish the final inequality, the left-hand side of (4) equals

$$\{\prod_{i=1}^{j} n_i/(n_i+1)\}\{1-\prod_{i=1}^{j}(1-1/n_i^2)\}$$

and the second factor of this is less than or equal to

$$\sum_{i=1}^{j} n_i^{-2} < \int_{n_j}^{\infty} x^{-2}\,dx + n_j^{-2} \le 2n_j^{-1}.$$

PROOF OF THEOREM 1. Note that

$$J((j,l);u^{\beta}) = \prod_{i\le j} n_i/(n_i+\beta), \quad \text{when} \quad n_j+\beta>0. \tag{5}$$

Expand $\phi(u_j)$ around $\hat{u}_j \equiv J((j,l);u)$ to see that for any $j \le k$

$$\begin{aligned} &|\phi(\hat{u}_j) - J((j,l);\phi)| \\ &\le \int |\phi(\hat{u}_j)-\phi(u_j)|\,d(u_1,\cdots,u_k) \qquad (6)\\ &= \int \tfrac{1}{2}(\hat{u}_j-u_j)^2|\phi''(\Delta u_j+(1-\Delta)\hat{u}_j)|\,d(u_1,\cdots,u_k) \end{aligned}$$

where $0<\Delta\le 1$, and the integrals here and throughout the remainder of the proof are over the set $\{0\le u_1\le\cdots\le u_k\le 1\}$. It follows from (3) that $|\phi''(\Delta u_j+(1-\Delta)\hat{u}_j)| \le K(u_j^{-\beta}+\hat{u}_j^{-\beta})$ for some $K<\infty$ and $\beta<5/2$. Thus (6) is bounded by a constant times

$$\hat{u}_j^{-\beta}\int (u_j-\hat{u}_j)^2\,d(u_1,\cdots,u_k) + \int (u_j-\hat{u}_j)^2 u_j^{-\beta}\,d(u_1,\cdots,u_k). \tag{7}$$

As in the lemma it follows from (5) that

$$\begin{aligned} \int (u_j-\hat{u}_j)^2\,d(u_1,\cdots,u_k) &= \prod_{i\le j}\frac{n_i}{n_i+2} - \hat{u}_j^2 \\ &= \hat{u}_j^2\left[\prod_{i\le j}\left\{1+\frac{1}{n_i(n_i+2)}\right\}-1\right] \\ &\le (2e^2/n_j)\hat{u}_j^2, \quad \text{for} \quad n_j\ge 1. \end{aligned}$$

Thus the first term in (7) is less than a constant times $(n_j)^{-1}\hat{u}_j^{2-\beta}$. It will now be shown that this is true for the second term. Making use of (5), the second term of (7) equals

$$\hat{u}_j^2\left(\prod_{i\le j}\frac{n_i}{n_i-\beta}\right)-2\hat{u}_j\left(\prod_{i\le j}\frac{n_i}{n_i+1-\beta}\right)+\prod_{i\le j}\frac{n_i}{n_i+2-\beta}$$

$$\begin{aligned}(8)\qquad &=\exp\left\{\sum_{i\le j}2\log\left(\frac{n_i}{n_i+1}\right)+\log\left(\frac{n_i}{n_i-\beta}\right)\right\}\\ &\quad -2\exp\left\{\sum_{i\le j}\log\left(\frac{n_i}{n_i+1}\right)+\log\left(\frac{n_i}{n_i+1-\beta}\right)\right\}\\ &\quad +\exp\left\{\sum_{i\le j}\log\left(\frac{n_i}{n_i+2-\beta}\right)\right\}.\end{aligned}$$

From $\log(1+x)=x+O(x^2)$, it follows that for $n_j \gtrsim 6$ this is

$$\begin{aligned}&=\exp\left\{\sum_{i\le j}-\frac{2}{n_i+1}+\frac{\beta}{n_i+\beta}+O(n_i^{-2})\right\}\\ &\quad -2\exp\left\{\sum_{i\le j}-\frac{1}{n_i+1}+\frac{\beta-1}{n_i+1-\beta}+O(n_i^{-2})\right\}\\ &\quad +\exp\left\{\sum_{i\le j}\frac{\beta-2}{n_i+2-\beta}+O(n_i^{-2})\right\}\\ &=\left[\exp\left\{\sum_{i\le j}\frac{\beta-2}{n_i+1}\right\}\right][\exp\{\textstyle\sum_{i\le j}O(n_i^{-2})\}-2\exp\{\textstyle\sum_{i\le j}O(n_i^{-2})\}\\ &\qquad\qquad +\exp\{\textstyle\sum_{i\le j}O(n_i^{-2})\}]\\ &=[\exp\{(\beta-2)\textstyle\sum_{i\le j}(n_i+1)^{-1}\}]\{O(\textstyle\sum_{i\le j}n_i^{-2})\}.\end{aligned}$$

Now $\exp\{-\sum_{i\le j}(n_i+1)^{-1}\}=\hat{u}_j(1+O(n_j^{-1}))$ and so (8) is bounded by a constant times $n_j^{-1}\hat{u}_j^{2-\beta}$, as required. It is easily checked that $\phi_1$ also satisfies (3) and a similar argument can be used to bound the difference between $\phi_1(J(R_{in}; u))$ and $J(R_{in}; \phi_1)$. Thus, if we let $R(t)$ equal the rank of the largest observation less than or equal to $t$ and $N(t)$ denotes the number of (censored or uncensored) observations greater than or equal to $t$,

$$\begin{aligned}n^{1/2}\Bigg|&\int_{N(t)\ge 6}\{\phi_0(J(R(t); u))-J(R(t); \phi_0)\}\,d\hat{F}_0^z\\ &+\int_{N(t)\ge 6}\{\phi_1(J(R(t); u))-J(R(t); \phi_1)\}\,d\hat{F}_1^z\Bigg|\\ &\le(\text{Const})\{\max|z_i|\}n^{1/2}\int\frac{(\hat{F}_T^M(t))^{2-\beta}\,d\hat{F}}{n\hat{F}}\end{aligned}$$

which tends to zero as $n\to\infty$ if $\beta\le 2$. If $\beta>2$, then this is bounded by a constant times

$$n^{-1/2} \int_{N(t)\geq 6} \hat{F}^{1-\beta}\, d\hat{F} \to 0, \quad \text{as} \quad n \to \infty$$

since $\hat{F}_T^M \geq \hat{F}$. The finite number of terms when $N(t) < 6$ are easily seen to be negligible. Since lim inf $n^{-1}\text{Var}(\mathscr{T}) > 0$, it follows that $(\mathscr{T} - \mathscr{T}^*)/\text{Var}^{1/2}(\mathscr{T}) \to 0$, as required.

PROOF OF THEOREM 2. Since $\hat{F}_T^{KM} \leq \hat{F}_T \leq \hat{F}_T^M$, we need only establish the equivalence of the forms with $\hat{F}_T^{KM}$ and $\hat{F}_T^M$. By (3) and the lemma there exists a $K < \infty$ such that

$$\begin{aligned}
&n^{1/2} \int |\phi_0(\hat{F}_T^M) - \phi_0(\hat{F}_T^{KM})|\, d\hat{F}_0^z \\
&\leq \{\max|z_i|\}Kn^{1/2} \int |\hat{F}_T^M - \hat{F}_T^{KM}|\, (\hat{F}_T^{KM})^{-(1+\alpha)}\, d\hat{F}, \quad \text{for some} \quad \alpha < \frac{1}{2} \\
&\leq (\text{const})\{\max|z_i|\}n^{-1/2} \int (\hat{F}_T^{KM})^{-\alpha}(\hat{F})^{-1}\, d\hat{F} \\
&\leq (\text{const})\{\max|z_i|\}n^{-1/2} \int (\hat{F})^{-(1+\alpha)}\, d\hat{F} \\
&\leq (\text{const})\{\max|z_i|\}n^{-3/2} \textstyle\sum_{i=1}^n (i/n)^{-(1+\alpha)} \to 0, \quad \text{as} \quad n \to \infty.
\end{aligned}$$

A similar argument shows that

$$n^{1/2} \int |\phi_1(\hat{F}_T^M) - \phi_1(\hat{F}_T^{KM})|\, d\hat{F}_1^z \to 0, \quad \text{as} \quad n \to \infty,$$

which completes the proof.

**3. Asymptotic normality and efficiency.** Another type of rank test used for censored data consists of weighted sums of the differences between observed and "conditionally expected values" of the covariate $z_i$ at each uncensored observation. Such statistics are of the general form

$$\tilde{\mathscr{T}} = (1/n) \textstyle\sum_{j=1}^n (1 - \varepsilon_i)(z_i - \bar{z}_i)\Phi(\hat{F}_T(X_i)) \tag{9}$$

where

$$\bar{z}_i = \textstyle\sum_{j=1}^n z_j I_{\{X_j \geq X_i\}} / \sum_{j=1}^n I_{\{X_j \geq X_i\}}$$

is the average value of the covariates for those "persons at risk" at the time of the observation of $X_i$.

Mehrotra, Michalek, and Mihalko (1982) have shown that if $\Phi(u) = \phi_0(u) - \phi_1(u)$ and $\Phi(\hat{F}_T(X_i))$ is replaced by the conditional expectation $E(\Phi \mid R_{in})$, then (9) is equal to Prentice's linear rank test with score function $\phi$. The methods of the previous section can be adapted to show that the use of approximate scores does not asymptotically affect the statistic, so that (2) and (9) are asymptotically equivalent under the conditions of Theorem 1. If we define $\tilde{\mathscr{T}}(t)$ as at (9) except that the summation is only over terms with $X_i < t$, then, when the $\{T_i\}$ are i.i.d. and under general assumptions on the censoring mechanism, $\tilde{\mathscr{T}}(t)$ is a square-integrable local martingale with respect to the $\sigma$-field $\mathscr{F}(t^-)$ generated by all observations less than $t$ and the indicator function of $\{\text{some } X_i = t\}$. Rebolledo's (1980) results can then be used (cf. Gill, 1980, and Anderson, Borgan, Gill and Keiding, 1982) to establish asymptotic normality. In particular it is not difficult to establish the following:

THEOREM 3. *Under the conditions of Theorem* 1, *and the additional assumptions that the* $\{T_i\}$ *are i.i.d. with survival function* $F_T$ *and that a random censorship model holds, i.e.* $X_i = \min(T_i, C_i)$ *for* $\{C_i\}$ *independent* (*not necessarily identically distributed*) *and independent of the* $\{T_i\}$, *then as* $n \to \infty$

$$n^{1/2}\mathscr{T}_n/\sigma_n \to \mathscr{N}(0, 1)$$

*where*

$$\sigma_n^2 = \int \Phi^2(F(t))\mathrm{Var}(z \mid \mathscr{F}(t^-))\, dF_0,$$

$$\mathrm{Var}(z \mid \mathscr{F}(t^-)) = F_C^{z^2}(t)/F_C(t) - (F_C^z(t)/F_C(t))^2,$$

$$F_0(t) = E(\hat{F}_0(t)), \quad F_C(t) = (1/n)\textstyle\sum_{i=1}^n P(C_i > t)$$

$$F_C^z(t) = (1/n)\textstyle\sum_{i=1}^n z_i P(C_i > t), \quad \text{etc.}$$

*The variance* $\sigma_n^2$ *can be consistently estimated by*

$$(1/n)\textstyle\sum_{i=1}^n (1 - \varepsilon_i)\Phi^2(\hat{F}_T(X_i))\mathrm{Var}(z_i)$$

*where*

$$\mathrm{Var}(z_i) = \textstyle\sum_{j=1}^n (z_j - \bar{z}_i)^2 I_{\{X_j \ge X_i\}}/\sum_{j=1}^n I_{\{X_j \ge X_i\}}.$$

A contiguity argument can be used to extend these results to regression alternatives. In particular if $T$ has a continuously differentiable density function $f$ and survival function $F_T$, $T_i = T + bw_i$ and $b \sim n^{-1/2}$, then $n^{1/2}(\mathscr{T} - \mu_n)/\sigma_n \to \mathscr{N}(0, 1)$, where

$$\mu_n = \int \Phi(F_T(t))\, [dF_0^z(t)/dF_0(t) - F^z(t)/F(t)]\, dF_0(t).$$

($F(t) = E\hat{F}(t)$, etc). Under the assumptions of Theorem 1 and this additional assumption on $f$, $\partial\mu_n/\partial b$ and $\sigma_n$ are uniformly continuous in $b$ for $b$ in a neighborhood of zero and some calculation shows that the efficacy of $\mathscr{T}$ is given by

$$e_{\mathscr{T}} = \lim_{n\to\infty}(\partial\mu_n/\partial b \mid_{b=0})^2/\sigma_n^2$$

$$= \lim_{n\to\infty} \frac{\left(\displaystyle\int_0^1 \Phi(u)\Phi^*(u)\left[F_C^{wz}(v) - \frac{F_C^w(v)F_C^z(v)}{F_C(v)}\right]_{v=F_T^{-1}(u)} du\right)^2}{\displaystyle\int_0^1 \Phi^2(u)\left[F_C^{z^2}(v) - \frac{(F_C^z(v))^2}{F_C(v)}\right]_{v=F_T^{-1}(u)} du},$$

provided the limit exists. Here $\Phi^*(u) = \phi_0^*(u) - \phi_1^*(u)$ where $\phi_0^*$ and $\phi_1^*$ are generated from the score function $\phi^* = f'(F_T^{-1}(u))/f(F_T^{-1}(u))$ and $F_C^{wz}(v) = (1/n) \sum_{i=1}^n w_i z_i P(C_i > v)$. As anticipated by Prentice (1978), the efficacy is maximized by taking $z_i = w_i$ and $\Phi = \Phi^*$. However these tests are not fully efficient with respect to their parametric analogues unless the $\{C_i\}$ are i.i.d. In that case the efficacy factors into two terms:

$$e_{\mathscr{T}} = \beta_1^2 \beta_2^2$$

where

$$\beta_1^2 = \lim_{n\to\infty} \frac{\sum_{i=1}^n (z_i - \bar{z})(w_i - \bar{w})}{\sum_{i=1}^n (z_i - \bar{z})^2}$$

provided the limit exists, and

$$\beta_2^2 = \left(E \int_0^1 \bar{\phi}(u, C)\bar{\phi}^*(u, C)\, du\right)^2 \Big/ E \int \bar{\phi}^2(u, C)\, du,$$

where $\bar{\phi}(u, C)$ is the censored influence curve defined by

$$\bar{\phi}(u, C) = \begin{cases} \phi_0(u), & u \geq F_T(C) \\ \phi_1(F(C)), & u < F_T(C) \end{cases}$$

and $\bar{\phi}^*$ is defined accordingly in terms of $\phi^*(u) = f'(F^{-1}(u))/f(F^{-1}(u))$. In this form, these expressions lead to formulae for asymptotic relative efficiencies which are natural generalizations of those found in Hájek and Sĭdák (1967, Chapter VII), the only difference being that one now correlates the censored influence functions $\bar{\phi}$ and $\bar{\phi}^*$ instead of the original score functions $\phi$ and $\phi^*$.

REMARKS. (i) For the two-sample case, these formulae are equivalent to those obtained by Gill (1980) and Leurgans (1984) and provide an alternative interpretation of their results in terms of the classical (uncensored) theory of rank tests.

(ii) It should be possible to weaken the condition that the $\{z_i\}$ are bounded. This would be important for example when the $\{z_i\}$ are i.i.d. random variables drawn from some unbounded distribution. The condition that $\phi''$ exists is probably also superfluous. It would also be nice to handle $\phi$ functions with a finite number of jump discontinuities, as in the median test. However, all the commonly used tests, e.g. the logrank test ($\phi = -1 - \log t$), the generalized Wilcoxon test ($\phi = 1 - 2t$) and Harrington and Fleming's (1982) intermediate family of tests ($\phi = \alpha - (1 + \alpha)t^\alpha$, $0 < \alpha \leq 1$) are easily seen to be covered.

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