## Smooth Goodness-of-Fit Tests in Hazard-Based Models

by

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#### 1. Practical Problem

- Right-censored survival data for lung cancer patients from Gatsonis, Hsieh and Korwar (1985).
- Survival times (in months): n = 86 with 23 right-censored.

 $\begin{array}{l} 0.99, \, 1.28, \, 1.77, \, 1.97, \, 2.17, \, 2.63, \, 2.66, \, 2.76, \, 2.79, \, 2.86, \, 2.99, \, 3.06, \, 3.15, \\ 3.45, \, 3.71, \, 3.75, \, 3.81, \, 4.11, \, 4.27, \, 4.34, \, 4.40, \, 4.63, \, 4.73, \, 4.93, \, 4.93, \, 5.03, \\ 5.16, \, 5.17, \, 5.49, \, 5.68, \, 5.72, \, 5.85, \, 5.98, \, 8.15, \, 8.26, \, 8.48, \, 8.61, \, 9.46, \, 9.53, \\ 10.05, \, 10.15, \, 10.94, \, 10.94, \, 11.04+, \, 11.24, \, 11.63, \, 12.26, \, 12.65, \, 12.78, \\ 13.18, \, 13.47, \, 13.53+, \, 13.96, \, 14.23+, \, 14.65+, \, 14.88, \, 14.91+, \, 15.05, \\ 15.31, \, 15.47+, \, 16.13, \, 16.46, \, 16.49+, \, 17.05+, \, 17.28+, \, 17.45, \, 17.61, \\ 17.88+, \, 17.97+, \, 18.20, \, 18.37, \, 18.83+, \, 19.06, \, 19.55+, \, 19.58+, \, 19.75+ \\ 19.78+, \, 19.95+, \, 20.04+, \, 20.24+, \, 20.70, \, 20.73+, \, 21.55+, \, 21.98+, \, 22.54, \\ 23.36 \end{array}$ 

• Probability histograms of the Complete and Censored Values.



• Product-limit estimator and best-fitting exponential survivor function.



• Question: Did the survival data come from the family of exponential distributions? Or was it from the family of Weibull distributions?

- Another Data Set: Times to withdrawal (in hours) of 171 car tires in Davis and Lawrance (1989, *Scand. J. Statist.*) from a car-tire testing study, with withdrawal either due to failure or right-censoring.
- The pneumatic tires were subjected to laboratory testing by rotating each tire against a steel drum until either failure [actually, there were several competing causes] or removal (right-censoring).

• Plots of the product-limit estimator, best-fitting exponential, and best-fitting Weibull distribution.



- Question: Is this failure-time car tires data consistent with an underlying Weibull distribution?
- Both situations point to a **goodness-of-fit** problem with right-censored data.

## 2. Densities and Hazards

• T = a positive-valued continuous failure-time variable, e.g.,

 $\diamond$  time-to-failure of a mechanical or electronic system

- $\diamond$  time-to-occurrence of an event
- $\diamond$  survival time of a patient in a clinical trial
- f(t) = density function of T.

$$f(t)\Delta t \approx \mathbf{P}\left\{T \in [t, t + \Delta t)\right\}.$$

- $F(t) = \mathbf{P}\{T \le t\}$  = distribution function
- $\bar{F}(t) = 1 F(t) =$ survivor function
- $\lambda(t) = f(t)/\bar{F}(t) =$  hazard rate function.

$$\lambda(t)\Delta t \approx \mathbf{P}\left\{T \in [t, t + \Delta t) | T \ge t\right\}.$$

- $\Lambda(t) = \int_0^t \lambda(w) dw = -\log[\bar{F}(t)] = (\text{cumulative}) \text{ hazard function}$
- Equivalences:

$$ar{F}(t) = \exp\{-\Lambda(t)\}$$
  
 $f(t) = \lambda(t) \exp\{-\Lambda(t)\}$ 

- Two Common Examples:
  - $\diamond$  Exponential:

$$f(t;\eta) = \eta \exp\{-\eta t\}$$
$$\bar{F}(t;\eta) = \exp\{-\eta t\}$$
$$\lambda(t;\eta) = \eta$$
$$\Lambda(t;\eta) = \eta t$$

 $\diamond$  Two-Parameter Weibull:

$$\begin{split} f(t;\alpha,\eta) &= (\alpha\eta)(\eta t)^{\alpha-1} \exp\{-(\eta t)^{\alpha}\}\\ \bar{F}(t;\alpha,\eta) &= \exp\{-(\eta t)^{\alpha}\}\\ \lambda(t;\alpha,\eta) &= (\alpha\eta)(\eta t)^{\alpha-1}\\ \Lambda(t;\alpha,\eta) &= (\eta t)^{\alpha} \end{split}$$

 $\diamond$  Qualitative Aspects from Plots of Hazards



Weibull Hazard Plots

## 3. A Pitch for Hazard-Based Modeling

- Advantages of specifying models via hazards:
  - ♦ Vantage point in density modeling: Time origin.
  - Vantage point in hazard modeling: 'Present, together with infor-mation that accumulated in the past.'
  - ♦ Qualitative aspects (e.g., IFR, or bath-tub) can be incorporated.
  - Facilitates incorporation of dynamic evolution of system or component.
  - ♦ Likelihood construction natural via product integrals.
  - ♦ Adapts well in the presence of right-censored or truncated data.
  - Conducive to modeling with point processes (popularized by Aalen;
     Andersen and Gill; etc.).

- Theory to be presented applicable to more general models, but will only consider the following hazard-based models.
  - ♦ **IID Model:**  $T_1, T_2, ..., T_n$  IID with common unknown hazard rate function  $\lambda(t)$ . Observable vectors:

$$(Z_1,\delta_1),(Z_2,\delta_2),\ldots,(Z_n,\delta_n)$$

with

$$\delta_i = 1 \Rightarrow T_i = Z_i$$
$$\delta_i = 0 \Rightarrow T_i > Z_i.$$

♦ **Cox PH Model** (also Andersen and Gill Model):

$$(T_1, X_1), (T_2, X_2), \dots, (T_n, X_n)$$

with

$$\lambda_{T|X}(t|X) = \lambda(t) \exp\{\beta^{t}X\}$$

 $\lambda(\cdot)$  an unknown hazard rate function, and  $\beta$  a regression coefficient vector. Observable vectors:

$$(Z_1, \delta_1, X_1), (Z_2, \delta_2, X_2), \dots, (Z_n, \delta_n, X_n)$$

with

$$\delta_i = 1 \Rightarrow T_i = Z_i$$
$$\delta_i = 0 \Rightarrow T_i > Z_i.$$

### 4. Problems and Issues

• Problem (Goodness-of-Fit): Given

$$\{(Z_i,\delta_i), i=1,2,\ldots,n\}$$

in the IID model, or

$$\{(Z_i, \delta_i, X_i), i = 1, 2, \dots, n\}$$

in the Cox model, decide whether

$$\lambda(\cdot) \in \mathcal{C} = \{\lambda_0(\cdot; \eta) : \eta \in \Upsilon\}$$

where  $\eta$  is a nuisance parameter vector.

 $\diamond \mathcal{C}$  could be: Exponential, Weibull, Gamma; or IFRA class.

♦ Importance?

- Previous works on GOF problem with censored data: Akritas (88, JASA), Hjort (90, AS), Hollander and Peña (92, JASA), Li and Doss (93, AS), and others.
- $\diamond$  Generalize Pearson's:

$$\chi_P^2 = \sum \frac{(O_j - \hat{E}_j)^2}{\hat{E}_j}?$$

 $\diamond$  Difficulty:  $O_j$ 's not computable with censored data. Also, any optimality properties of existing tests?

- Problem (Model Validation): Given {(Z<sub>i</sub>, δ<sub>i</sub>), i = 1, 2, ..., n} or {(Z<sub>i</sub>, δ<sub>i</sub>, X<sub>i</sub>), i = 1, 2, ..., n}, how to assess model assumptions (e.g., IID assumption, model structure)?
  - ♦ Unit Exponentiality Property (UEP)

$$T \sim \Lambda(\cdot) \Rightarrow \Lambda(T) \sim \text{EXP}(1)$$

♦ IID model: If  $\Lambda_0(\cdot)$  is the true hazard function, then with  $R_i^0 = \Lambda_0(Z_i)$ ,

$$(R_1^0, \delta_1), (R_2^0, \delta_2), \dots, (R_n^0, \delta_n)$$

is a right-censored sample from EXP(1).

 $\diamond$  Since  $\Lambda_0(\cdot)$  is not known, the  $R_i^0$ 's are estimated by  $R_i$ 's with

$$R_i = \Lambda(Z_i), \quad i = 1, 2, \dots, n,$$

 $\hat{\Lambda}(\cdot)$  is an estimator of  $\Lambda_0(\cdot)$  based on the  $(Z_i, \delta_i)$ 's.

- ◊ Idea:  $(R_i, \delta_i)$ 's assumed to form an approximate right-censored sample from EXP(1), so to validate model, test whether  $(R_i, \delta_i)$ 's is a right-censored sample from EXP(1).
- ♦ **Question:** How good is this *approximation*, even in the limit???

 $\diamond$  For Cox PH model, the analogous expressions for  $R_i^0$  and  $R_i$  are:

$$R_i^0 = \Lambda_0(Z_i) \exp\{\beta X_i\}, \quad i = 1, 2, \dots, n;$$
$$R_i = \hat{\Lambda}(Z_i) \exp\{\hat{\beta} X_i\}, \quad i = 1, 2, \dots, n.$$

- \*  $\hat{\Lambda}(\cdot)$  is an estimator of  $\Lambda_0(\cdot)$  [e.g., Aalen-Breslow estimator]. \*  $\hat{\beta}$  is an estimator of  $\beta$  [partial likelihood MLE].
- $\diamond R_i^0$ 's are true generalized residuals (Cox and Snell (68, JRSS)).
- $\diamond R_i$ 's are estimated generalized residuals.
- ♦ Generalized residuals are analogs of linear model residuals:

## "(Observed Value) minus (Fitted Value)."

◊ Question: Effects of substituting estimators for the unknown parameters, even when the estimators are consistent??

### 5. Class of Smooth GOF Tests

• Convert observed data

$$(Z_1, \delta_1, X_1), (Z_2, \delta_2, X_2), \dots, (Z_n, \delta_n, X_n)$$

into stochastic processes.

• For i = 1, 2, ..., n, and  $t \ge 0$ , let

$$N_i(t) = I\{Z_i \le t, \delta_i = 1\};$$
$$Y_i(t) = I\{Z_i \ge t\}.$$

•

$$A_i(t;\lambda(\cdot),\beta) = \int_0^t Y_i(w)\lambda(w) \exp\{\beta^{t}X_i\}dw;$$
$$M_i(t;\lambda(\cdot),\beta) = N_i(t) - A_i(t;\lambda(\cdot),\beta).$$

• If  $\lambda_0(\cdot)$  and  $\beta_0$  are the *true* parameters,

$$M^0(t) = (M_1(t; \lambda_0(\cdot), \beta_0), \dots, M_n(t; \lambda_0(\cdot), \beta_0))$$

are orthogonal sq-int zero-mean martingales with predictable quadratic variation processes

$$\langle M_i^0, M_i^0 \rangle(t) = A_i(t; \lambda_0(\cdot), \beta_0).$$

• Problem: Test

 $H_0: \lambda(\cdot) \in \mathcal{C} = \{\lambda_0(\cdot; \eta) : \eta \in \Upsilon\}$  versus  $H_1: \lambda(\cdot) \notin \mathcal{C}.$ 

 Idea: If λ<sub>0</sub>(·) is the true hazard rate function, then under H<sub>0</sub> there is some η<sub>0</sub> ∈ Υ such that

$$\lambda_0(\cdot) = \lambda_0(\cdot;\eta_0).$$

• Define

$$\kappa(t;\eta) = \log\left[rac{\lambda_0(t)}{\lambda_0(t;\eta)}
ight].$$

- $\mathcal{K}$  = collection of such { $\kappa(\cdot; \eta) : \eta \in \Upsilon$  }.
- Basis set (e.g., trigonometric, polynomial, wavelet, etc.) for  $\mathcal{K}$ :

$$\Psi = \{\psi_1(\cdot;\eta), \psi_2(\cdot;\eta), \ldots\};$$
  

$$\kappa(t;\eta) = \sum_{j=1}^{\infty} \theta_j \psi(t;\eta) = \theta' \Psi.$$

• For smoothing order K, approximate  $\kappa(\cdot; \eta)$  by

$$\kappa(t;\eta) = \sum_{j=1}^{K} \theta_j \psi(t;\eta) = \theta'_K \Psi_K.$$

• Equivalently,

$$\lambda_0(t) \approx \lambda_0(t;\eta) \exp\left\{\sum_{j=1}^K \theta_j \psi(t;\eta)\right\}.$$

• Define:

$$\mathcal{C}_{K} = \left\{ \lambda_{K}(\cdot; \theta, \eta) = \lambda_{0}(\cdot; \eta) \exp\left\{ \sum_{j=1}^{K} \theta_{j} \psi_{j}(\cdot; \eta) \right\} : \quad \theta_{K} \in \Re^{K}; \eta \in \Upsilon \right\}.$$

• Embedding:  $H_0 \subset \mathcal{C}_K$ .

• Goodness-of-Fit Tests: Score tests for

$$H_0: \theta_K = 0, \beta \in \mathcal{B}$$
 versus  $H_1: \theta_K \neq 0, \beta \in \mathcal{B}$ .

- Tests introduced in Peña (1998, JASA; 1998, AS).
- Since score tests, they possess local optimality properties.
- Likelihood function: obtained via product integration.
- Score function for  $\theta_K$  at  $\theta_K = 0$ :

$$Q(\eta,\beta) = \sum_{i=1}^{n} \int_{0}^{\tau} \Psi_{K}(\eta) \left\{ \mathrm{d}N_{i} - Y_{i}\lambda_{0}(\eta) \exp\{\beta^{\mathrm{t}}X_{i}\}\mathrm{d}t \right\}$$

- Not a statistic since  $\eta$  and  $\beta$  are unknown.
- Plug-in estimators for  $\eta$  and  $\beta$  under the restriction  $\theta_K = 0$ .
- Estimate  $\beta$  by  $\hat{\beta}$  which solves

$$S(\beta) \equiv \sum_{i=1}^{n} \int_{0}^{\tau} [X_i - E(\beta)] dN_i = 0;$$
$$E(t, \beta) = \frac{S^{(1)}(t, \beta)}{S^{(0)}(t, \beta)};$$
$$S^{(m)}(t, \beta) = \sum_{i=1}^{n} X_i^{\otimes m} Y_i \exp\{\beta^{t} X_i\}, \quad m = 0, 1, 2$$

• Estimate  $\eta$  by  $\hat{\eta}$  which solves

$$egin{aligned} R(\eta, \hat{eta}) &\equiv \sum_{i=1}^n \int_0^ au 
ho(\eta) \left\{ \mathrm{d}N_i - Y_i \lambda_0(\eta) \exp\{\hat{eta}^{\mathrm{t}} X_i\} \mathrm{d}t 
ight\} = 0; \ &
ho(t, \eta) = rac{\partial}{\partial \eta} \log \lambda_0(t, \eta). \end{aligned}$$

• Test statistic:

$$S_K = \frac{1}{n} \left\{ \hat{Q}^{\mathrm{t}} \right\} \left\{ \hat{\Xi}^{-1} \right\} \left\{ \hat{Q} \right\},\,$$

with

$$\hat{Q} = Q(\hat{\eta}, \hat{\beta}) = \sum_{i=1}^{n} \int_{0}^{\tau} \Psi_{K}(\hat{\eta}) \left\{ \mathrm{d}N_{i} - Y_{i}\lambda_{0}(\hat{\eta}) \exp\{\hat{\beta}^{\mathrm{t}}X_{i}\} \mathrm{d}t \right\}$$

- $\hat{\Xi}$  is an estimator of the limiting covariance matrix of  $\frac{1}{\sqrt{n}}\hat{Q}$ .
- $S_K$  is a function of the generalized residuals  $(R_1, \delta_1), \ldots, (R_n, \delta_n)$ .

## 6. Asymptotics

• Proposition: If the parameters are known,

$$\frac{1}{\sqrt{n}} \begin{bmatrix} Q(\eta_0, \beta_0) \\ R(\eta_0, \beta_0) \\ S(\beta_0) \end{bmatrix} \xrightarrow{\mathrm{d}} N \begin{bmatrix} \begin{pmatrix} 0 \\ 0 \\ 0 \end{pmatrix}, \Sigma = \begin{pmatrix} \Sigma_{11} & \Sigma_{12} & 0 \\ \Sigma_{21} & \Sigma_{22} & 0 \\ 0 & 0 & \Sigma_{33} \end{pmatrix} \end{bmatrix},$$

 $\mathbf{SO}$ 

$$\frac{1}{\sqrt{n}}Q(\eta_0,\beta_0) \stackrel{\mathrm{d}}{\longrightarrow} N(0,\Sigma_{11}).$$

• Theorem: With estimated parameters,

$$\frac{1}{\sqrt{n}}\hat{Q} \equiv \frac{1}{\sqrt{n}}Q(\hat{\eta},\hat{\beta}) \xrightarrow{\mathrm{d}} N_K(0, \ \Xi)$$

where

$$\Xi = \Sigma_{11} - \Sigma_{12} \Sigma_{22}^{-1} \Sigma_{21} + (\Delta_1 - \Sigma_{12} \Sigma_{22}^{-1} \Delta_2) \Sigma_{33}^{-1} (\Delta_1 - \Sigma_{12} \Sigma_{22}^{-1} \Delta_2)^{\mathrm{t}}.$$

• **Proof:** Relies on Rebolledo's MCT.

#### 7. Effects of the Plug-In Procedure

 $\diamond$  From

$$\Xi = \Sigma_{11} - \Sigma_{12} \Sigma_{22}^{-1} \Sigma_{21} + (\Delta_1 - \Sigma_{12} \Sigma_{22}^{-1} \Delta_2) \Sigma_{33}^{-1} (\Delta_1 - \Sigma_{12} \Sigma_{22}^{-1} \Delta_2)^{\mathrm{t}},$$

replacing  $(\eta, \beta)$  by  $(\hat{\eta}, \hat{\beta})$  to obtain the statistic  $\hat{Q}$  has **no asymptotic** effect if

$$\Sigma_{12} = 0 \quad \text{and} \quad \Delta_1 = 0,$$

since  $\Sigma_{11}$  is the limiting covariance for  $\frac{1}{\sqrt{n}}Q(\eta_0,\beta_0)$ .

- $\diamond$  Essence of "adaptiveness:" it does not matter that nuisance parameters  $(\eta, \beta)$  are unknown in  $Q(\eta, \beta)$  since replacing them by their estimators does **not** make the asymptotic distribution of  $Q(\hat{\eta}, \hat{\beta})$  different from  $Q(\eta_0, \beta_0)$ .
- $\diamond \Sigma_{12} = 0$  is an orthogonality condition between  $\Psi_K(\eta_0)$  and  $\rho(\eta_0)$ .
- $\diamond \Delta_1 = 0$  is an orthogonality condition between  $\rho(\eta_0)$  and  $e(\eta_0, \beta_0)$ .
- $\diamond$  Orthogonality defined in a Hilbert space whose inner product is

$$\langle f,g\rangle = \int_0^\tau fg \nu_0(\mathrm{d}t),$$

with

$$u_0(A) = \int_A s^{(0)}(\eta_0, \beta_0) \lambda_0(\eta_0) \mathrm{d}t;$$
 $s^{(0)}(t; \eta_0, \beta_0) = \mathrm{plim} \frac{1}{n} S^{(0)}(t; \eta_0, \beta_0).$ 

- $\diamond$  Can we always choose  $\Psi_K$  to satisfy orthogonality conditions? Yes, via a Gram-Schmidt process ... but hard to implement!
- ◊ If orthogonality conditions are not satisfied, substituting (η̂, β̂) for (η<sub>0</sub>, β<sub>0</sub>) in Q(η<sub>0</sub>, β<sub>0</sub>) impacts on the asymptotic distribution of Q̂, even if the estimators are consistent.
- $\diamond$  Second term in  $\Xi$ : effect of estimating  $\eta$  by  $\hat{\eta}$ .
- $\diamond$  Third term in  $\Xi$ : effect of estimating  $\beta$  by the partial MLE  $\hat{\beta}$ .
- $\diamond$  Estimating  $\beta$  by  $\hat{\beta}$  leads to an increase in variance because this estimator is less efficient than the full MLE of  $\beta$ .
- ◊ Ignoring effect on variance could have dire consequences in the goodness-of-fit testing.
- ◊ If overall effect is a variance reduction, ignoring effect may result in a highly conservative test and lead into concluding model appropriateness when model is inappropriate.

# 8. Form of Test Procedure

• Recall:

$$\hat{Q} = Q(\hat{\eta}, \hat{\beta}) = \sum_{i=1}^{n} \int_{0}^{\tau} \Psi_{K}(\hat{\eta}) \left\{ \mathrm{d}N_{i} - Y_{i}\lambda_{0}(\hat{\eta}) \exp\{\hat{\beta}^{\mathrm{t}}X_{i}\}\mathrm{d}t \right\}.$$

• Estimating Limiting Covariance Matrix,  $\Xi$ : With

$$\begin{split} \hat{\Sigma} &= \frac{1}{2n} \sum_{i=1}^{n} \int_{0}^{\tau} \begin{bmatrix} \Psi_{K}(\hat{\eta}) \\ \rho(\hat{\eta}) \\ X_{i} - E(\hat{\beta}) \end{bmatrix}^{\otimes 2} \left\{ \mathrm{d}N_{i} + Y_{i}\lambda_{0}(\hat{\eta}) \exp\{\hat{\beta}^{\mathrm{t}}X_{i}\}\mathrm{d}t \right\}; \\ \hat{\Delta}_{1} &= \frac{1}{2n} \sum_{i=1}^{n} \int_{0}^{\tau} \Psi_{K}(\hat{\eta}) E(\hat{\beta})^{\mathrm{t}} \left\{ \mathrm{d}N_{i} + Y_{i}\lambda_{0}(\hat{\eta}) \exp\{\hat{\beta}^{\mathrm{t}}X_{i}\}\mathrm{d}t \right\}; \\ \hat{\Delta}_{2} &= \frac{1}{2n} \sum_{i=1}^{n} \int_{0}^{\tau} \rho(\hat{\eta}) E(\hat{\beta})^{\mathrm{t}} \left\{ \mathrm{d}N_{i} + Y_{i}\lambda_{0}(\hat{\eta}) \exp\{\hat{\beta}^{\mathrm{t}}X_{i}\}\mathrm{d}t \right\}; \end{split}$$

an estimator of  $\Xi$  is

$$\hat{\Xi} = \hat{\Sigma}_{11} - \hat{\Sigma}_{12}\hat{\Sigma}_{22}^{-1}\hat{\Sigma}_{21} + (\hat{\Delta}_1 - \hat{\Sigma}_{12}\hat{\Sigma}_{22}^{-1}\hat{\Delta}_2)\hat{\Sigma}_{33}^{-1}(\hat{\Delta}_1 - \hat{\Sigma}_{12}\hat{\Sigma}_{22}^{-1}\hat{\Delta}_2)^{t}.$$

• Asymptotic  $\alpha$ -Level Test:

Reject  $H_0: \lambda(\cdot) \in \mathcal{C}; \beta \in \mathcal{B}$  whenever

$$S_K = \frac{1}{n} \left\{ \hat{Q}^{\mathrm{t}} \right\} \left\{ \hat{\Xi}^- \right\} \left\{ \hat{Q} \right\} \ge \chi^2_{K^*;\alpha};$$

 $\hat{\Xi}^-$  = Moore-Penrose generalized inverse of  $\hat{\Xi}$  and  $K^* = \operatorname{rank}(\hat{\Xi})$ .

## 9. Choices for $\Psi_K$

- Partition  $[0, \tau]$ :  $0 = a_0 < a_1 < ... < a_{K-1} < a_K = \tau$
- Let

$$\Psi_K(t) = \left[ I_{[0,a_1]}(t), I_{(a_1,a_2]}(t), \dots, I_{(a_{K-1},\tau]}(t) \right]^t.$$

• Then

$$\hat{Q} = [O_1 - E_1, O_2 - E_2, \dots, O_K - E_K]^{t}$$

where

$$O_{j} = \sum_{i=1}^{n} \int_{a_{j-1}}^{a_{j}} \mathrm{d}N_{i}(t)$$
$$E_{j} = \sum_{i=1}^{n} \int_{a_{j-1}}^{a_{j}} Y_{i}(t) \exp\{\hat{\beta}^{\mathrm{t}}X_{i}\}\lambda_{0}(t;\hat{\eta})\mathrm{d}t.$$

- $O_j$ 's are observed frequencies.
- $E_j$ 's are *estimated* dynamic expected frequencies.
- Extends Pearson's statistic, but 'counts are in a dynamic fashion.'
- Resulting test statistic **not** of form

$$\sum_{j=1}^{K} \frac{(O_j - E_j)^2}{E_j}$$

because correction terms in variance destroys diagonal nature of covariance matrix.

- If adjustments are ignored, distribution is **not** chi-squared.
- Procedure extends Akritas (1988) and Hjort (1990).

### • Example:

- Consider no covariates (so 
$$X_i = 0$$
);

 $-\mathcal{C} = \{\lambda_0(t;\eta) = \eta\}, \text{ that is, Exponential distribution.}$ 

• For 
$$j = 1, 2, ..., K$$
,

$$O_j = \sum_{i=1}^n \int_{a_{j-1}}^{a_j} dN_i(t);$$
$$E_j = \hat{\eta} \sum_{i=1}^n \int_{a_{j-1}}^{a_j} Y_i(t) dt;$$
$$\hat{\eta} = \frac{\sum_{i=1}^n \int_0^\tau dN_i(t)}{\sum_{i=1}^n \int_0^\tau Y_i(t) dt} = \frac{\text{Number of events}}{\text{Total exposure}}.$$

• Resulting test statistic:

$$S_K = E_{\bullet} \left[ \mathbf{p} - \hat{\pi} \right]^{\mathrm{t}} \left[ \mathrm{Dg}(\hat{\pi}) - \hat{\pi} \hat{\pi}^{\mathrm{t}} \right]^{-} \left[ \mathbf{p} - \hat{\pi} \right],$$

where

$$E_{\bullet} = \sum_{j=1}^{K} E_j;$$
  
$$\mathbf{p} = \frac{1}{E_{\bullet}} (O_1, O_2, \dots, O_K)^{\mathrm{t}};$$
  
$$\hat{\pi} = \frac{1}{E_{\bullet}} (E_1, E_2, \dots, E_K)^{\mathrm{t}}.$$

•  $E_{\bullet} \neq n$ .

• Appropriate degrees-of-freedom for the statistic is K-1 since  $\mathbf{1}^{t}\hat{\pi} = 1$ .

#### • Polynomial-type:

$$\Psi_K(t;\eta) = \left[1, \ \Lambda_0(t;\eta), \ \Lambda_0(t;\eta)^2, \ \dots, \ \Lambda_0(t;\eta)^{K-1}\right]^{t}.$$

• Resulting  $\hat{Q}$  vector has components  $\hat{Q}_j, j = 1, 2, \dots, K$  given by

$$\hat{Q}_{j} = \sum_{i=1}^{n} \exp\{-(j-1)\hat{\beta}X_{i}\} \int_{0}^{\tau} w^{j-1} \{ \mathrm{d}N_{i}^{R}(w) - Y_{i}^{R}(w)\mathrm{d}w\},\$$

where, with

$$R_i = \Lambda_0(Z_i; \hat{\eta}) \exp\{\hat{\beta}X_i\}$$

being the estimated residuals,

$$N_i^R(w) = I\{R_i \le w; \delta_i = 1\};$$
$$Y_i^R(w) = I\{R_i \ge w\}.$$

- $N_i^R$ 's and  $Y_i^R$ 's are generalized residual processes.
- Resulting test generalizes test proposed by Hyde's (mid '70's).
- Other Choices: Total-time-on-test ... this leads to a generalization of a test proposed by Barlow, Bartholomew, Brunk and Bremmer (1972) ... good for IFR alternatives.

## 10. Levels of Tests for Different K's

• Polynomial:  $\Psi_K(t;\eta) = (1, \Lambda_0(t;\eta), \dots, \Lambda_0(t;\eta)^{K-1})^t$ 

K ∈ {1, 2, 3, 4}; censoring proportion ∈ {25%, 50%} and true failure time model were exponential and 2-Weibull. # of Reps = 2000

Null Dist.		Exponential $(\eta)$			
Parameters		$\eta = 2$		$\eta = 5$	
% Uncensored		75%	50%	75%	50%
Level		5%	5%	5%	5%
n	K				
	2	4.65	6.65	5.00	5.60
50	3	5.45	5.50	4.35	4.95
	4	6.55	5.50	5.10	5.85
	5	6.40	5.00	5.20	4.20
	2	4.90	4.75	4.45	4.35
100	3	4.55	4.35	4.65	4.25
	4	5.70	5.30	5.10	4.80
	5	5.75	4.90	5.30	4.75
N	ull Dist.		Weibu	$\mathrm{ll}(lpha,\eta)$	
Nu Pa:	ull Dist. rameters	$(\alpha,\eta) =$	Weibu $(2,1)$	$\frac{\mathrm{ll}(\alpha,\eta)}{(\alpha,\eta)} =$	= (3, 2)
Nu Pai % U	ull Dist. rameters ncensored	$(\alpha, \eta) = 75\%$	Weibu $(2, 1)$ 50%	$ \begin{array}{c} \mathrm{ll}(\alpha,\eta) \\ (\alpha,\eta) = \\ 75\% \end{array} $	=(3,2) 50%
Nu Pa: % U	ull Dist. rameters ncensored Level	$(\alpha, \eta) = 75\%$ $5\%$	Weibu = (2, 1) 50% 5%	$ \begin{array}{c} \mathrm{ll}(\alpha,\eta) \\ (\alpha,\eta) = \\ \hline 75\% \\ \hline 5\% \end{array} $	(3, 2) 50% 5%
Nu Pa: % U n	ull Dist. rameters ncensored Level K	$(\alpha, \eta) = 75\%$ 5%	Weibu = (2, 1) 50% 5%	$ \begin{array}{c} \mathrm{ll}(\alpha,\eta) \\ (\alpha,\eta) = \\ \hline 75\% \\ \hline 5\% \end{array} $	=(3,2) 50% 5%
Nu Pai % U n	all Dist. rameters ncensored Level K 2	$(\alpha, \eta) = 75\%$ 5% 4.30	Weibu = (2, 1) 50% 5% 4.80	$ \begin{array}{r} 11(\alpha,\eta) \\ (\alpha,\eta) = \\ 75\% \\ 5\% \\ 4.60 \end{array} $	(3, 2) 50% 5% 6.20
Nu Pa: % U n 50	all Dist. rameters ncensored Level K 2 3	$(\alpha, \eta) =$ $75\%$ $5\%$ $4.30$ $5.40$	Weibu = (2, 1) 50% 5% 4.80 5.15	$ \begin{array}{r} 11(\alpha,\eta) \\ (\alpha,\eta) = \\ 75\% \\ 5\% \\ 4.60 \\ 6.30 \end{array} $	(3, 2) 50% 5% 6.20 5.70
Nu Pa: % U n 50	all Dist. rameters ncensored Level <i>K</i> 2 3 4	$(\alpha, \eta) = 75\%$ 5% 4.30 5.40 4.80	Weibu = (2, 1) 50% 5% 4.80 5.15 4.80	$ \begin{array}{r} 11(\alpha,\eta) \\ (\alpha,\eta) = \\ 75\% \\ 5\% \\ \hline \\ 4.60 \\ 6.30 \\ 6.10 \end{array} $	(3, 2) 50% 5% 6.20 5.70 5.30
Nu Pa: % U n 50	all Dist.rametersncensoredLevel $K$ 2345	$(\alpha, \eta) = 75\%$ 5% 4.30 5.40 4.80 5.25	Weibu = (2, 1) 50% 5% 4.80 5.15 4.80 3.45	$ \begin{array}{r} \text{ll}(\alpha,\eta) \\ (\alpha,\eta) = \\ 75\% \\ 5\% \\ 4.60 \\ 6.30 \\ 6.10 \\ 6.70 \end{array} $	=(3,2) 50% 5% 6.20 5.70 5.30 4.60
Nu Pa: % U n 50	all Dist. rameters ncensored Level <i>K</i> 2 3 4 5 2	$(\alpha, \eta) = 75\%$ $5\%$ $4.30$ $5.40$ $4.80$ $5.25$ $3.90$	Weibul = (2, 1) 50% 5% 4.80 5.15 4.80 3.45 4.95	$ \begin{array}{r} 11(\alpha,\eta) \\ (\alpha,\eta) = \\ 75\% \\ 5\% \\ 4.60 \\ 6.30 \\ 6.10 \\ 6.70 \\ 4.20 \end{array} $	= (3, 2) 50% 5% 6.20 5.70 5.30 4.60 4.80
Nu Pa: % U n 50	$\begin{array}{c} \text{all Dist.} \\ \text{rameters} \\ \text{ncensored} \\ \text{Level} \\ \hline \\ \hline \\ \\ \hline \\ \\ \\ \\ \\ \\ \\ \\ \\ \\ \\ \\ $	$(\alpha, \eta) = 75\%$ $5\%$ $4.30$ $5.40$ $4.80$ $5.25$ $3.90$ $5.75$	Weibu: = (2, 1) 50% 5% 4.80 5.15 4.80 3.45 4.95 4.65	$ \begin{array}{r} 11(\alpha,\eta) \\ (\alpha,\eta) = \\ 75\% \\ 5\% \\ 4.60 \\ 6.30 \\ 6.10 \\ 6.70 \\ 4.20 \\ 5.15 \end{array} $	= (3, 2) $50%$ $5%$ $6.20$ $5.70$ $5.30$ $4.60$ $4.80$ $5.25$
Nu Pa: % U n 50	In the second stateand the second staterametersncensoredLevel $K$ 23452344	$(\alpha, \eta) = 75\%$ $5\%$ $4.30$ $5.40$ $4.80$ $5.25$ $3.90$ $5.75$ $5.55$	$\begin{array}{r} \text{Weibu}\\\hline\hline\\ (2,1)\\ 50\%\\ 5\%\\ \hline\\ 4.80\\ 5.15\\ 4.80\\ 3.45\\ 4.95\\ 4.65\\ 4.15\\ \end{array}$	$ \begin{array}{r} 11(\alpha,\eta) \\ (\alpha,\eta) = \\ 75\% \\ 5\% \\ 4.60 \\ 6.30 \\ 6.10 \\ 6.70 \\ 4.20 \\ 5.15 \\ 5.00 \end{array} $	(3, 2) 50% 5% 6.20 5.70 5.30 4.60 4.80 5.25 4.30

## 11. Power Function of Tests

Simulation Parameters: n = 100; 25% censoring.

**Legend:** Solid line (K = 2); Dots (K = 3); Short dashes (K = 4); Long dashes (K = 5).

Figure 1: Simulated Powers for Null: Exponential vs. Alt: 2-Weibull



Figure 2: Simulated Powers for Null: Exponential vs. Alt: 2-Gamma







#### Some Observations:

- Appropriate smoothing order K depends on alternative considered.
- Not always necessary to have large K!
- Tests based on  $S_3$  and  $S_4$  could serve as omnibus tests, at least for the models in these simulations.
- Calls for a formal method to dynamically determine K. Plan: to utilize information-based criteria to determine appropriate smoothing order.

#### 12. Back to the Lung Cancer Data

• Product-Limit Estimator (PLE) of survival curve together with confidence band, and the best fitting exponential survival curve.



• **Testing Exponentiality:** Values of test statistics using the polynomialtype specification, together with their *p*-values are:

$$S_2 = 1.92(p = .1661);$$
  $S_3 = 1.94(p = .3788);$   
 $S_4 = 7.56(p = .0561);$   $S_5 = 12.85(p = .0121).$ 

- Close to a constant function, but with high frequency terms.
- Testing Two-Parameter Weibull: Value of S<sub>3</sub>, together with its *p*-value, is

$$S_3 = 8.35 \quad (p = .0153)$$

Values of  $S_4$  and  $S_5$  both indicate rejection of two-parameter Weibull model.

## 13. Back to the Car Tire Example



Results for Testing Exponentiality

Summary of Results of Smooth Goodness-of-Fit Test Testing for Exponential Distribution

Input FileName: C:\Talks\TalkATUG\davislawrancecartiredata.txt
Output FileName: C:\Talks\TalkATUG\cartire.out

00000						
Sum of Delta_i = 150						
6185094769E-003						
Estimate of Mean = 243						
P-Value						
1.00000						
0.00000						
0.00000						
0.00000						
0.00000						

**Conclusion:** Results very consistent with the graphical display: the exponential model does not hold.

Results for Testing the Weibull Class Summary of Results of Smooth Goodness-of-Fit Test Testing for the Two-Parameter Weibull Distribution Using the Polynomial Specification for Psi Input FileName: C:\Talks\TalkATUG\davislawrancecartiredata.txt Output FileName: C:\Talks\TalkATUG\cartireWeibull.txt Sample Size = 171 # of Uncensored Values = 150.00000000000 3.41809555692639 Estimate of alpha = Estimate of eta = 4.085166006131305E-003 S\_k DF P-Value k 0.0000 1 0 1.00000 2 0.5013 1 0.47891 3 0.5550 2 0.75769 4 3 6.4203 0.09286 5 6.5183 4 0.16364

#### **Conclusions From Analysis**

- Cannot reject the hypothesis that the Weibull class holds for this car tire data.
- Notice in particular the p-values associated with the different smoothing order.
- Result of test consistent with the plots of the survivor estimates.

#### 14. Extensions and Open Problems

- When failure times are discrete or interval=censored. Paper with V. Nair now in draft form. New twist is that embedding of the discrete hazards is through their odds!
- How about if the failure times are of a mixed-type, i.e., with continuous and discrete components?
- Problem of adaptively determining the smoothing order to be addressed.
- Promising possibility for  $\Psi_{K}$ : Wavelets as Basis??!

#### 15. Conclusions

- Presented a formal approach for developing GOF tests with censored data, and extends in a more *natural* way Neyman's smooth gof tests (cf., Rayner and Best (1989), *Smooth Goodness of Fit Tests*).
- Resulting tests possess optimality conditions by virtue of being score tests.
- Able to see effects of estimating nuisance parameters: (Morale: Don't Ignore!)
- Potential problems when using generalized residuals in model validation: (Morale: Intuitive considerations may Fail!)