Midterm: STAT 570

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1. Consider an situation in which we are interested in the risk of death in the first 5 years of life (the under-5 mortality mortality risk, or U5MR) in each of 2n areas in two consecutive time periods. Consider a hypothetical situation in which a malaria prevention intervention is randomized across the areas, immediately after the first time periods. Areas indexed by  $i=1,\ldots,n$  are control areas, while areas  $i=n+1,\ldots,2n$  receiving the intervention.

In each area and each time period alive/dead status of  $M_{it}$  children are recorded, call the number dead  $D_{it}$  for i = 1, ..., 2n, t = 0, 1. Let

$$Y_{it} = \log\left(\frac{D_{it}/M_{it}}{1 - D_{it}/M_{it}}\right),\tag{1}$$

denote the logit of the U5MR in area i in period t, i = 1, ..., n, t = 0, 1.

Suppose the true model is given by

$$Y_{it} = \beta_0 + \alpha_i + \beta_1 x_{it} + \epsilon_{it}, \tag{2}$$

where  $\alpha_i \sim \mathcal{N}\left(0, \sigma_{\alpha}^2\right)$  are area-specific random effects and  $\epsilon_{it} \sim \mathcal{N}\left(0, \sigma_{\epsilon}^2\right)$ , represents measurement error, with  $\alpha_i$  and  $\epsilon_{it}$  independent,  $i = 1, \ldots, 2n, t = 0, 1$ . The covariate  $x_{it}$  is an indicator for the intervention so that  $x_{i0} = 0$  for  $i = 1, \ldots, 2n, x_{i1} = 0$  for  $i = 1, \ldots, n$ , and  $x_{i1} = 1$  for  $i = n + 1, \ldots, 2n$ .

We will consider three models for the child mortality data:

Follow-up model:  $Y_{i1} = \beta_0^{\dagger} + \beta_1^{\dagger} x_{i1} + \epsilon_{i1}^{\dagger}$ , for  $i = 1, \dots, 2n$ .

Change model:  $Z_i = Y_{i1} - Y_{i0} = \beta_0^* + \beta_1^* x_{i1} + \epsilon_i^*$ , for i = 1, ..., 2n.

Analysis for Covariance (ANCOVA) model:  $Y_{i1} = \beta_0^{\ddagger} + \gamma Y_{i0} + \beta_1^{\ddagger} x_{i1} + \epsilon_i^{\ddagger}$ , for  $i = 1, \dots, 2n$ .

(a) Carefully interpret  $\beta_1^{\dagger}$ ,  $\beta_1^{\star}$  and  $\beta_1^{\ddagger}$  in these models, and hence what each of  $\mathbb{E}\left[\hat{\beta}_1^{\dagger}\right]$ ,  $\mathbb{E}\left[\hat{\beta}_1^{\star}\right]$ , and  $\mathbb{E}\left[\hat{\beta}_1^{\dagger}\right]$  are unbiased estimators of.

Solution: Let's examine each case.

 $\beta_1^{\dagger}$ : Let  $Y_{:,1} = \begin{pmatrix} Y_{1,1} & \cdots & Y_{2n,1} \end{pmatrix}^{\mathsf{T}}$ . Let  $\beta = \begin{pmatrix} \beta_0 & \beta_1 \end{pmatrix}^{\mathsf{T}}$ . Let X be the  $2n \times 2$  matrix with 1s in the first column and  $x_{1,1}, \ldots, x_{2n,1}$  in the second column. We can write  $Y_{:,1} = X\beta + \alpha_i + \epsilon_{:,1}$ .

We have that

$$\hat{\beta}^{\dagger} = (X^{\mathsf{T}}X)^{-1} X^{\mathsf{T}}Y_{:,1} = (X^{\mathsf{T}}X)^{-1} X^{\mathsf{T}} (X\beta + \alpha + \epsilon_{:,1})$$

$$= \beta + (X^{\mathsf{T}}X)^{-1} X^{\mathsf{T}} (\alpha + \epsilon_{:,1})$$

$$\sim \mathcal{N} \left(\beta, \left(\sigma_{\alpha}^{2} + \sigma_{\epsilon}^{2}\right) (X^{\mathsf{T}}X)^{-1}\right), \tag{3}$$

so we'll obtain unbiased estimates of  $\beta$  with higher variance than if we had the correct model.

So,  $\beta_1^{\dagger}$  is the expected change in the logit of the U5MR after applying the treatment.

 $\beta_1^*$ : We have that  $Z_i = Y_{i1} - Y_{i0} = \beta_1 (x_{i1} - x_{i0}) + \epsilon_{i1} - \epsilon_{i0} = \beta_1 x_{i1} + (\epsilon_{i1} - \epsilon_{i0})$ . Solving for  $\hat{\beta}^*$ , we find

$$\hat{\beta}^{\star} = (X^{\mathsf{T}}X)^{-1} X^{\mathsf{T}} Z_{i} = (X^{\mathsf{T}}X)^{-1} X^{\mathsf{T}} \left( X \begin{pmatrix} 0 \\ \beta_{1} \end{pmatrix} + (\epsilon_{:,1} - \epsilon_{:,0}) \right)$$

$$= \begin{pmatrix} 0 \\ \beta_{1} \end{pmatrix} + (X^{\mathsf{T}}X)^{-1} X^{\mathsf{T}} (\epsilon_{:,1} - \epsilon_{:,0})$$

$$\sim \mathcal{N} \left( \begin{pmatrix} 0 \\ \beta_{1} \end{pmatrix}, 2\sigma_{\epsilon}^{2} (X^{\mathsf{T}}X)^{-1} \right), \tag{4}$$

so  $\hat{\beta}_1^{\star}$  is an unbiased estimate of  $\beta_1$ .

Thus,  $\beta_1^{\star}$  is again the expected change in the logit of the U5MR after applying the treatment.

 $\beta_1^{\ddagger}$ : Consider the different ways of writing  $Y_{i1}$ ,

$$Y_{i1} = \beta_0 + \alpha_i + \beta_1 x_{i1} + \epsilon_{i1}$$

$$= \beta_0^{\ddagger} + \gamma Y_{i0} + \beta_1^{\ddagger} x_{i1} + \epsilon_i^{\ddagger}$$

$$= \beta_0^{\ddagger} + \beta_1^{\ddagger} x_{i1} + \gamma (\beta_0 + \alpha_i + \epsilon_{i0}) + \epsilon_i^{\ddagger}$$
(5)

Define  $X^{\ddagger}$  to be the  $2n \times 3$  matrix with the first two columns being X and third column being  $Y_{:,0}$ .

Then, we have that

$$\begin{pmatrix} \hat{\beta}_0^{\dagger} \\ \hat{\beta}_1^{\dagger} \\ \hat{\gamma} \end{pmatrix} = \left( \left( X^{\dagger} \right)^{\mathsf{T}} X^{\dagger} \right)^{-1} \left( X^{\dagger} \right)^{\mathsf{T}} Y_{:,1}. \tag{6}$$

From Equation 5, note that

$$Y_{i1} - \gamma Y_{i0} = (1 - \gamma) \beta_0 + \beta_1 x_{i1} + (1 - \gamma) \alpha_i - \gamma \epsilon_{i0} + \epsilon_{i1}$$
$$= \beta_0^{\ddagger} + \beta_1^{\ddagger} X_{i1} + \epsilon_i^{\ddagger}.$$

We can estimate  $\gamma$  with Equation 15. Given  $\hat{\gamma}$ , the least squares estimate

for  $\beta^{\ddagger}$  is

$$\begin{pmatrix}
\hat{\beta}_{0}^{\dagger} \\
\hat{\beta}_{1}^{\dagger}
\end{pmatrix} \mid \hat{\gamma} = (X^{\mathsf{T}}X)^{-1} X^{\mathsf{T}} (Y_{:,1} - \hat{\gamma}Y_{:,0}) 
= \begin{pmatrix}
(1 - \hat{\gamma}) \beta_{0} \\
\beta_{1}
\end{pmatrix} + (X^{\mathsf{T}}X)^{-1} X^{\mathsf{T}} ((1 - \hat{\gamma}) \alpha_{i} + \hat{\gamma}\epsilon_{i0} + \epsilon_{i1}) 
\sim \mathcal{N} \left( \begin{pmatrix}
(1 - \hat{\gamma}) \beta_{0} \\
\beta_{1}
\end{pmatrix}, \left( (1 - \hat{\gamma})^{2} \sigma_{\alpha}^{2} + \hat{\gamma}^{2} \sigma_{\epsilon}^{2} + \sigma_{\epsilon}^{2} \right) (X^{\mathsf{T}}X)^{-1} \right).$$
(7)

Regardless of  $\hat{\gamma}$ ,  $\hat{\beta}_1^{\ddagger}$  is an unbiased estimate of  $\beta_1$ , for

$$\mathbb{E}\left[\hat{\beta}_{1}^{\ddagger}\right] = \mathbb{E}_{\hat{\gamma}}\left[\mathbb{E}\left[\hat{\beta}_{1}^{\ddagger} \mid \hat{\gamma}\right]\right] \mathbb{E}_{\hat{\gamma}}\left[\beta_{1}\right] = \beta_{1}$$

by law of total expectation.

All in all, we have that the expected value of the estimates

$$\mathbb{E}\left[\hat{\beta}_{1}^{\dagger}\right] = \mathbb{E}\left[\hat{\beta}_{1}^{\star}\right] = \mathbb{E}\left[\hat{\beta}_{1}^{\dagger}\right] = \beta_{1},\tag{8}$$

so  $\beta_1^{\dagger}$ ,  $\beta_1^{\star}$ ,  $\beta_1^{\dagger}$  can all be interpreted as the expected change in U5MR after applying the treatment.

(b) Evaluate var  $(\hat{\beta}_1^{\dagger})$ , var  $(\hat{\beta}_1^{\star})$ , and var  $(\hat{\beta}_1^{\dagger})$ . Comment on the efficiency of the estimators arising from each of the three models.

**Solution:** While Equation 8 tells us that the expectation of our estimators is the same, the variances are different.

 $\hat{\beta}_1^{\dagger}$ : We can compute the variance from Equation 3. First, we have that

$$X^{\mathsf{T}}X = \begin{pmatrix} 2n & \sum_{i=1}^{2n} x_{i1} \\ \sum_{i=1}^{2n} x_{i1} & \sum_{i=1}^{2n} x_{i1}^{2} \end{pmatrix} = \begin{pmatrix} 2n & n \\ n & n \end{pmatrix}$$

$$\implies (X^{\mathsf{T}}X)^{-1} = \frac{1}{n^{2}} \begin{pmatrix} n & -n \\ -n & 2n \end{pmatrix} = \frac{1}{n} \begin{pmatrix} 1 & -1 \\ -1 & 2 \end{pmatrix}. \tag{9}$$

Thus, we find that

$$\operatorname{var}\left(\hat{\beta}_{1}^{\dagger}\right) = \frac{2}{n} \left(\sigma_{\alpha}^{2} + \sigma_{\epsilon}^{2}\right). \tag{10}$$

 $\hat{\beta}_1^{\star}$ : Using Equations 4 and 9, we compute that

$$\operatorname{var}\left(\hat{\beta}_{1}^{\star}\right) = \frac{4}{n}\sigma_{\epsilon}^{2}.\tag{11}$$

 $\hat{\beta}_1^{\ddagger}$ : We use Equation 7 to compute the variance conditional in terms of  $\hat{\gamma}$ . First, we note that

$$\operatorname{var}\left(\hat{\beta}_{1}^{\ddagger}\right) = \left((1-\hat{\gamma})^{2} \sigma_{\alpha}^{2} + \hat{\gamma}^{2} \sigma_{\epsilon}^{2} + \sigma_{\epsilon}^{2}\right) (X^{\intercal} X)_{22}^{-1}$$

$$= \frac{2}{n} \left((1-\hat{\gamma})^{2} \sigma_{\alpha}^{2} + \hat{\gamma}^{2} \sigma_{\epsilon}^{2} + \sigma_{\epsilon}^{2}\right)$$

$$= \frac{2}{n} \left(\hat{\gamma}^{2} \left(\sigma_{\alpha}^{2} + \sigma_{\epsilon}^{2}\right) - 2\hat{\gamma}\sigma_{\alpha}^{2} + \sigma_{\alpha}^{2} + \sigma_{\epsilon}^{2}\right). \tag{12}$$

From Equations 10 and 11, whether the follow-up model or change model estimates  $\beta_1$  more efficiently depends on whether the variance of the random effect is larger than the random effect of the measurement error. When the variance of the random effect is larger  $(\sigma_{\alpha}^2 > \sigma_{\epsilon}^2)$ , var  $(\hat{\beta}_1^{\dagger}) < \text{var}(\hat{\beta}_1^{\dagger})$ , so the change model is more efficient. Otherwise if  $\sigma_{\alpha}^2 < \sigma_{\epsilon}^2$ , the follow-up model is more efficient.

The ANCOVA model is more interesting. From Equation 12, when  $\hat{\gamma} = 0$ , efficiency is the same as the follow-up model, and when  $\hat{\gamma} = 1$ , efficiency is the same as the change model. var  $(\hat{\beta}_1^{\ddagger})$  is a strictly convex function of  $\hat{\gamma}$  which is minimized at

$$\hat{\gamma}^* = \frac{\sigma_\alpha^2}{\sigma_\alpha^2 + \sigma_\epsilon^2}.\tag{13}$$

By the Gauss-Markov theorem that states that the ordinary least squares estimate gives the lowest variance estimate for unbiased estimators, we must have that  $\hat{\gamma} = \hat{\gamma}^*$ , so

$$\operatorname{var}\left(\hat{\beta}_{1}^{\ddagger}\right) = \frac{2}{n} \left( (1 - \hat{\gamma}^{*})^{2} \sigma_{\alpha}^{2} + (\hat{\gamma}^{*})^{2} \sigma_{\epsilon}^{2} + \sigma_{\epsilon}^{2} \right)$$

$$= \frac{2}{n} \left( \frac{\sigma_{\alpha}^{2} \sigma_{\epsilon}^{2}}{\sigma_{\alpha}^{2} + \sigma_{\epsilon}^{2}} + \sigma_{\epsilon}^{2} \right) \leq \frac{2}{n} \left( \max \left( \sigma_{\alpha}^{2}, \sigma_{\epsilon}^{2} \right) + \sigma_{\epsilon}^{2} \right), \quad (14)$$

which results in  $\hat{\beta}_1^{\ddagger}$  being a more efficient estimator than both  $\hat{\beta}_1^{\dagger}$  and  $\hat{\beta}_1^{\star}$ . The behavior of  $\hat{\gamma}$  will be investigated more fully in the next two parts.

(c) Obtain an expression for  $\hat{\gamma}$ , in as simple a form as you can find.

**Solution:** From Equation 7, we have that

$$\hat{\gamma} = \left( \left( \left( X^{\ddagger} \right)^{\mathsf{T}} X^{\ddagger} \right)^{-1} \left( X^{\ddagger} \right)^{\mathsf{T}} Y_{:,1} \right)_{3} \tag{15}$$

$$= \sum_{k=1}^{3} \left( \left( X^{\ddagger} \right)^{\mathsf{T}} X^{\ddagger} \right)_{3k}^{-1} \left( \left( X^{\ddagger} \right)^{\mathsf{T}} Y_{:,1} \right)_{k}$$

$$= -n \frac{\sum_{i=1}^{n} Y_{i0}}{\det \left( (X^{\ddagger})^{\mathsf{T}} X^{\ddagger} \right)} \sum_{i=1}^{2n} Y_{i1} + n \frac{\sum_{i=1}^{n} Y_{i0} - \sum_{i=n+1}^{2n} Y_{i0}}{\det \left( (X^{\ddagger})^{\mathsf{T}} X^{\ddagger} \right)} \sum_{i=n+1}^{2n} Y_{i1}$$

$$+ \frac{n^{2}}{\det \left( (X^{\ddagger})^{\mathsf{T}} X^{\ddagger} \right)} \sum_{i=1}^{2n} Y_{i0} Y_{i1}$$

$$= \frac{n}{\det \left( (X^{\ddagger})^{\mathsf{T}} X^{\ddagger} \right)} \left( n \sum_{i=1}^{2n} Y_{i0} Y_{i1} - \sum_{i=1}^{n} Y_{i0} \sum_{i=n+1}^{n} Y_{i0} \sum_{i=n+1}^{2n} Y_{i1} \right),$$

where  $n/\det\left(\left(X^{\ddagger}\right)^{\mathsf{T}}X^{\ddagger}\right)$  can be obtained from Equation 16. One can also write Equation 15 in terms of empirical variance estimates as in Equation 19.

$$\frac{1}{n} \det\left(\left(X^{\dagger}\right)^{\mathsf{T}} X^{\dagger}\right) = n \sum_{i=1}^{2n} Y_{i0}^{2} - \left(\sum_{i=1}^{n} Y_{i0}\right)^{2} - \left(\sum_{i=1}^{n} Y_{i0}\right) \left(\sum_{i=n+1}^{2n} Y_{i0}\right) - \left(\sum_{i=n+1}^{2n} Y_{i0}\right) \left(2 \sum_{i=n+1}^{2n} Y_{i0} - \sum_{i=1}^{2n} Y_{i0}\right) \\
= n \sum_{i=1}^{2n} Y_{i0}^{2} - \left(\sum_{i=1}^{n} Y_{i0}\right)^{2} - \left(\sum_{i=n+1}^{2n} Y_{i0}\right)^{2}.$$
(16)

(d) On the basis of the previous question, or otherwise, give intuitive explanations for the efficiency results in Part 1b.

**Solution:** Denote the MLE estimates of the covariance between  $Y_{i0}$  and  $Y_{i1}$  without and with the intervention by

$$\hat{\operatorname{cov}}(Y_{i0}, Y_{i1} \mid x_{i1} = 0) = \frac{1}{n} \sum_{i=1}^{n} Y_{i0} Y_{i1} - \left(\frac{1}{n} \sum_{i=1}^{n} Y_{i0}\right) \left(\frac{1}{n} \sum_{i=1}^{n} Y_{i1}\right) 
\hat{\operatorname{cov}}(Y_{i0}, Y_{i1} \mid x_{i1} = 1) = \frac{1}{n} \sum_{i=n+1}^{2n} Y_{i0} Y_{i1} - \left(\frac{1}{n} \sum_{i=n+1}^{2n} Y_{i0}\right) \left(\frac{1}{n} \sum_{i=n+1}^{2n} Y_{i1}\right),$$
(17)

respectively. Similarly, we can denote the MLE of the variances of  $Y_{i0}$  without and with the intervention by

$$var(Y_{i0} \mid x_{i1} = 0) = \frac{1}{n} \sum_{i=1}^{n} Y_{i0}^{2} - \left(\frac{1}{n} \sum_{i=1}^{n} Y_{i0}\right)^{2}$$

$$var(Y_{i0} \mid x_{i1} = 1) = \frac{1}{n} \sum_{i=n+1}^{2n} Y_{i0}^{2} - \left(\frac{1}{n} \sum_{i=n+1}^{2n} Y_{i0}\right)^{2},$$
(18)

respectively. Substituting Equations 17 and 18 into the numerator and denominator of Equation 15, respectively, we can write

$$\hat{\gamma} = \frac{\hat{\text{cov}}(Y_{i0}, Y_{i1} \mid x_{i1} = 0) + \hat{\text{cov}}(Y_{i0}, Y_{i1} \mid x_{i1} = 1)}{\hat{\text{var}}(Y_{i0} \mid x_{i1} = 0) + \hat{\text{var}}(Y_{i0} \mid x_{i1} = 1)},$$
(19)

so we can interpret  $\gamma$  as the overall autocorrelation between  $Y_{i0}$  and  $Y_{i1}$ . Based on the true model, we can compute

$$\operatorname{var}(Y_{i0}) = \operatorname{var}(\epsilon_{i0}) + \operatorname{var}(\alpha_{i}) = \sigma_{\epsilon}^{2} + \sigma_{\alpha}^{2}$$

$$\operatorname{cov}(Y_{i0}, Y_{i1} \mid x_{i1} = 0) = \operatorname{cov}(Y_{i0}, Y_{i1} \mid x_{i1} = 1)$$

$$= \mathbb{E}[(\alpha_{i} + \epsilon_{i0})(\alpha_{i} + \epsilon_{i1})]$$

$$= \operatorname{var}(\alpha_{i}) = \sigma_{\alpha}^{2},$$

so the expected value of  $\hat{\gamma}$  is

$$\mathbb{E}\left[\hat{\gamma}\right] \approx \frac{\sigma_{\alpha}^2}{\sigma_{\alpha}^2 + \sigma_{\epsilon}^2} \tag{20}$$

for large n by Stutsky's theorem, which is the value in Equation 13 that minimizes the variance.