Session 3
The proportional odds model and the Mann-Whitney test

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MRC Hubs for Trials Methodology Research
Edinburgh Hub

MRC Hubs for Trials Methodology Research
North West Hub

3.1 A unified approach to inference

Data (assumed here to be discrete): **x** = $(x_1, \ldots, x_n)$

A single unknown parameter: θ

Likelihood:

$$L(\theta; \mathbf{x}) = \text{“probability” of } \mathbf{x}, \text{ given } \theta$$

Log-likelihood:

$$\ell(\theta) = \log L(\theta; \mathbf{x})$$

Suppose that θ is small (it might be a treatment effect)

Taylor's expansion:

$$\begin{aligned}\ell(\theta) &= \ell(0) + \theta\ell'(0) + \tfrac{1}{2}\theta^2\ell''(0) + O(\theta^3) \\ &\approx \text{const.} + \theta Z - \tfrac{1}{2}\theta^2 V\end{aligned}$$

where

$Z = \ell'(0)$, efficient score

$V = -\ell''(0)$, Fisher's information

For large samples and small θ

$$Z \sim N(\theta V, V)$$

approximately *(Scharfstein et al., 1997)*

This is the basis for many common statistical tests:

- Pearson's Chi-squared test
- Armitage's trend test
- The logrank test
- The Mann-Whitney (or Wilcoxon) test

and it leads to asymptotically efficient methods

Estimation of θ

1. Maximum likelihood estimate

$$\hat{\theta}$$

where $\ell'(\hat{\theta}) = 0$

2. Estimate based on score statistics

$$\frac{Z}{V}$$

which has variance $\frac{1}{V}$

Hypothesis testing

To test the null hypothesis of H_0: $\theta = 0$

1. Likelihood ratio test

$$W = -2\log\left\{L(0)/L(\hat{\theta})\right\} \sim \chi_1^{\,2}$$

2. Score test

$$\frac{Z^2}{V} \sim \chi_1^{\,2}$$

3. Wald test

$$\left\{\frac{\hat{\theta}}{\text{s.e.}\left(\hat{\theta}\right)}\right\}^2 \sim \chi_1^{\,2}$$

For likelihoods with nuisance parameters: ϕ

Replace log-likelihood with profile log-likelihood

$$\ell(\theta, \phi) \approx \ell(\theta, \hat{\phi}(\theta))$$

where $\hat{\phi}(\theta)$ is the maximum likelihood estimate of ϕ, given the value of θ

$\ell(\theta, \hat{\phi}(\theta))$ is a function of θ only

Example: Binary data

	Control Group	Treated Group	Total
Success	s_C	s_T	s
Failure	f_C	f_T	f
Total	n_C	n_T	n

Probability of success: p_C and p_T on C and T respectively

$$\theta = \log_e\left(\frac{p_T(1-p_C)}{p_C(1-p_T)}\right) \qquad \text{(log-odds ratio)}$$

The unconditional likelihood of θ (comparison of binomial observations) leads to (see Session 2)

	Control Group	Treated Group	Total
Success	s_C	s_T	s
Failure	f_C	f_T	f
Total	n_C	n_T	n

$$Z = \frac{s_T f_C - s_C f_T}{n} \qquad V' = \frac{n_C n_T s f}{n^3}$$

The conditional likelihood of θ given s successes in total (hypergeometric distribution) leads to

	Control Group	**Treated Group**	**Total**
Success	s_C	s_T	s
Failure	f_C	f_T	f
Total	n_C	n_T	n

$$Z = \frac{s_T f_C - s_C f_T}{n} \qquad V = \frac{n_C n_T s f}{n^2 (n-1)}$$

3.2 Analysis via dichotomisation

Head injury data from Example 1

GOS at 3 months	GCS on entry		Total
	3-5	6-8	
1. Good Recovery	73	219	292
2. Moderate Disability	55	118	173
3. Severe Disability	79	66	145
4. Vegetative State	37	10	47
5. Dead	358	92	450
Total	602	505	1107

1. Success is Good GOS

	GCS on entry		Total
	3-5	6-8	
Success	**73**	**219**	292
Failure	**529**	**286**	815
Total	602	505	1107

θ = log odds of success for (GCS = 6-8) versus (GCS = 3-5)

$Z_1 = 85.8,\qquad V_1 = 53.4$

Estimate of $\theta = Z_1/V_1 = 1.61$

Score test: $Z_1^2/V_1 = 138.1$ (c.f. χ^2 on 1 df)

2. Success is Good or Moderate GOS

	GCS on entry		Total
	3-5	**6-8**	
Success	**128**	**337**	465
Failure	**474**	**168**	642
Total	602	505	1107

θ = log odds of success for (GCS = 6-8) versus (GCS = 3-5)

$Z_2 = 124.9$, $V_2 = 67.0$

Estimate of $\theta = Z_2/V_2 = 1.87$

Score test: $Z_2^2/V_2 = 232.8$ (c.f. χ^2 on 1 df)

3. Failure is Vegetative or Dead

	GCS on entry		Total
	3-5	6-8	
Success	**207**	**403**	610
Failure	**395**	**102**	497
Total	602	505	1107

θ = log odds of success for (GCS = 6-8) versus (GCS = 3-5)

$Z_3 = 124.7, \qquad V_3 = 68.0$

Estimate of $\theta = Z_3/V_3 = 1.83$

Score test: $Z_3^2/V_3 = 228.7$ (c.f. χ^2 on 1 df)

4. Failure is Dead

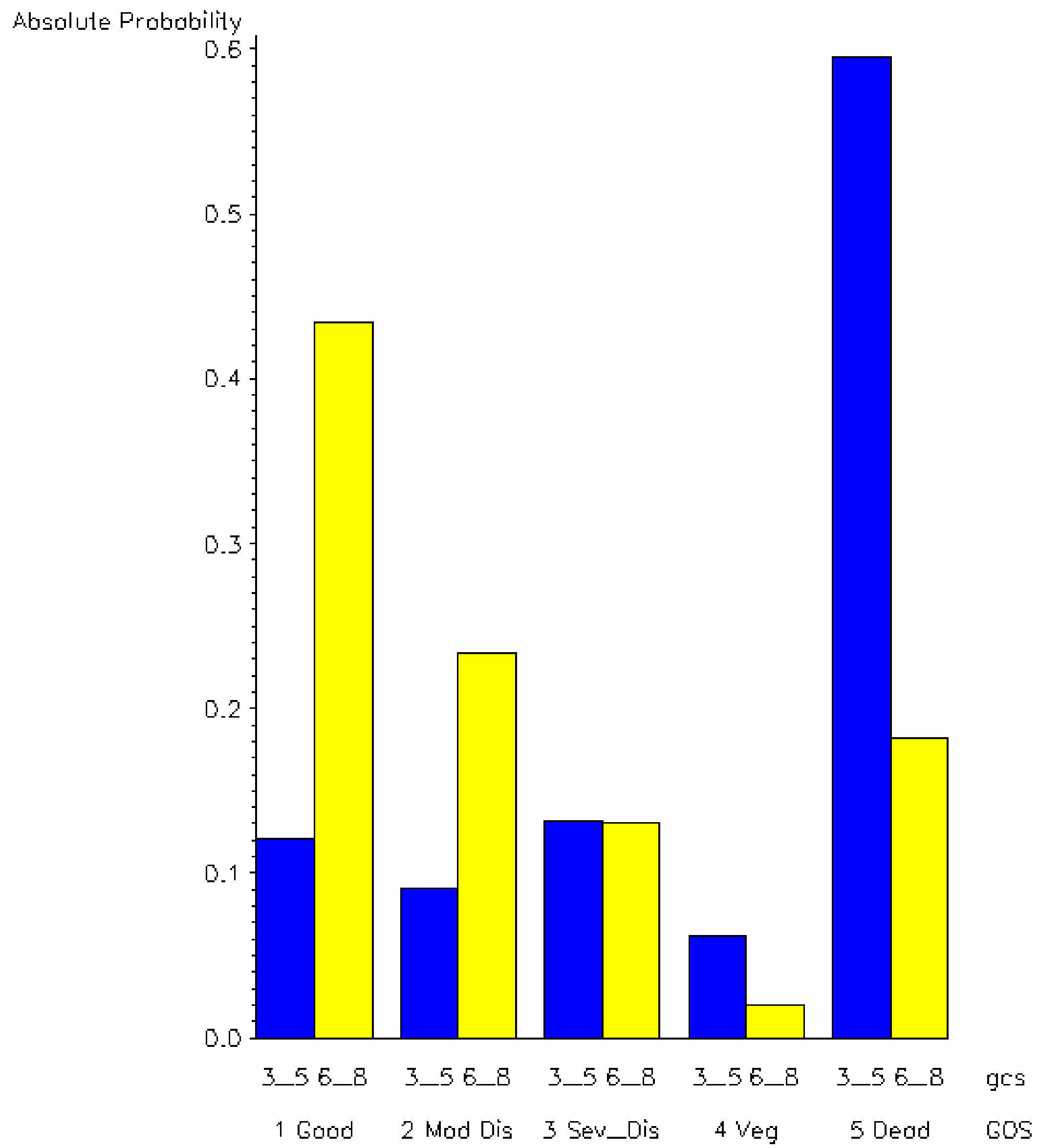
	GCS on entry		Total
	3-5	6-8	
Success	**224**	**413**	657
Failure	**358**	**92**	450
Total	602	505	1107

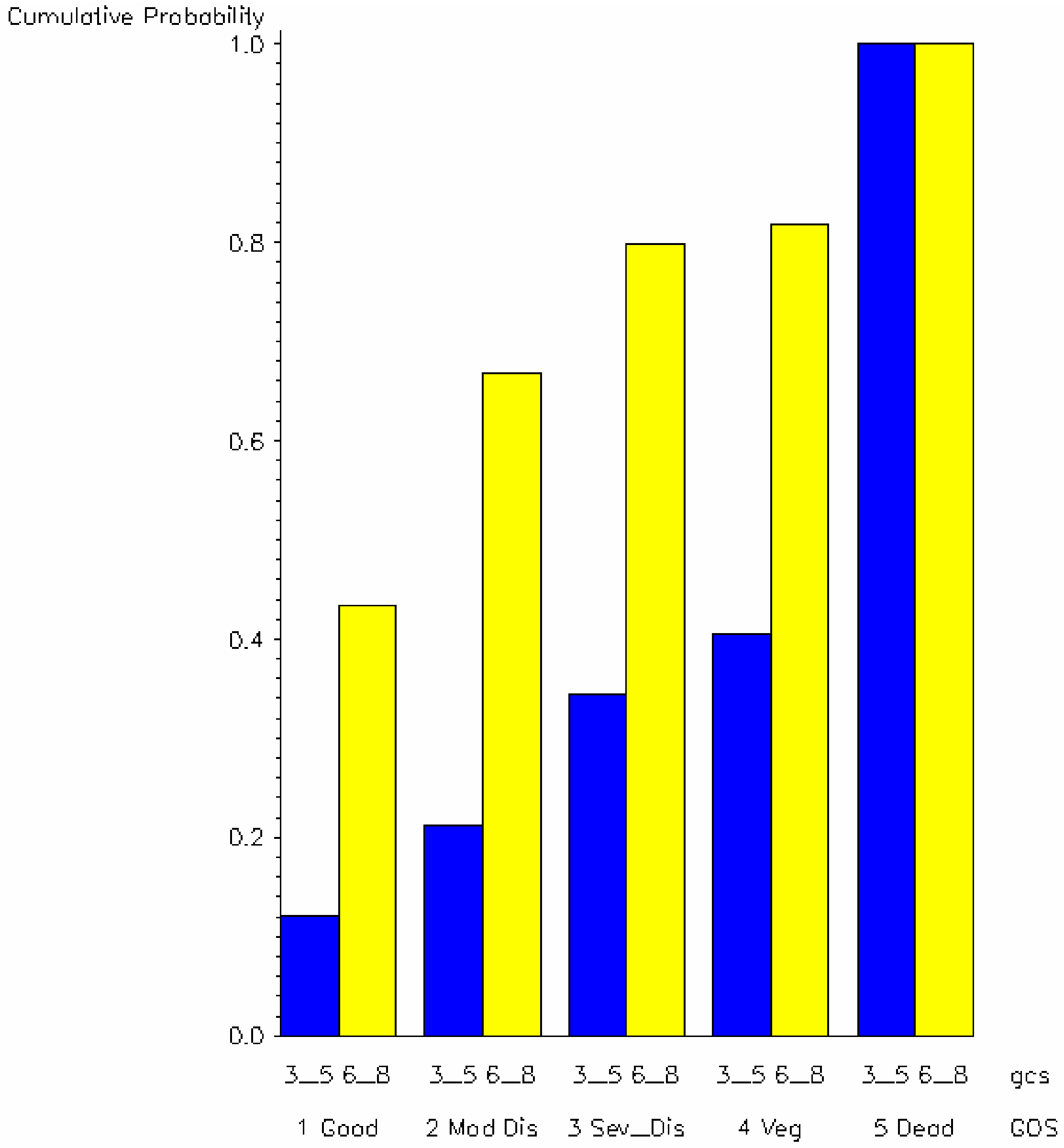
θ = log odds of success for (GCS = 6-8) versus (GCS = 3-5)

$Z_4 = 113.3$, $V_4 = 66.3$

Estimate of $\theta = Z_4/V_4 = 1.71$

Score test: $Z_4^2/V_4 = 193.6$ (c.f. χ^2 on 1 df)


Absolute Probability
0.6
0.5
0.4
0.3
0.2
0.1
0.0
3_5 6_8
3_5 6_8
3_5 6_8
3_5 6_8
3_5 6_8
gcs
1 Good
2 Mod Dis
3 Sev_Dis
4 Veg
5 Dead
GOS


Cumulative Probability
1.0
0.8
0.6
0.4
0.2
0.0
3_5 6_8
3_5 6_8
3_5 6_8
3_5 6_8
3_5 6_8
gcs
1 Good
2 Mod Dis
3 Sev_Dis
4 Veg
5 Dead
GOS

- Analyses each indicate that GCS 6-8 is preferable to GCS 3-5

- Magnitude of advantage, on the log-odds ratio scale, is consistent

How can these four analyses be combined?

3.3 Proportional odds

Notation

Category	Control Group	Treated Group	Total
C_1	n_{1C}	n_{1T}	n_1
C_2	n_{2C}	n_{2T}	n_2
⋮	⋮	⋮	⋮
C_m	n_{mC}	n_{mT}	n_m
Total	n_C	n_T	n

Let

$$L_{kT} = n_{1T} + \ldots + n_{(k-1)T}, \qquad U_{kT} = n_{(k+1)T} + \ldots + n_{mT}$$
$$L_{kC} = n_{1C} + \ldots + n_{(k-1)C}, \qquad U_{kC} = n_{(k+1)C} + \ldots + n_{mC}$$

Thus, if Success is $\{C_1, \ldots, C_k\}$, the derived 2×2 table is

	Control Group	Treated Group	Total
Success	$L_{(k+1)C}$	$L_{(k+1)T}$	s
Failure	U_{kC}	U_{kT}	f
Total	n_C	n_T	n

Let

p_{kC} = $P(C_k$; Control Group)

Q_{kC} = $P(C_k$ or Better; Control Group)
= $p_{1C} + \ldots + p_{kC}$, $k = 1, \ldots, m$;

so that $Q_{mC} = 1$

p_{kT} and Q_{kT} are defined similarly for treated group

and

$$\theta_k = \log\left\{\frac{Q_{kT}(1-Q_{kC})}{Q_{kC}(1-Q_{kT})}\right\} \qquad k = 1, \ldots, m-1$$

θ_k is the log-odds ratio of Success

where Success is $\{C_1, \ldots, C_k\}$

The **proportional odds assumption** is

$$\theta_1 = \theta_2 = \ldots = \theta_{m-1} = \theta$$

The common value, θ, is a measure of the advantage of being in the Treated Group

$$\theta \begin{cases} > & 0 \quad \text{Treated Group better} \\ = & 0 \quad \text{no difference} \\ < & 0 \quad \text{Treated Group worse} \end{cases}$$

Score and information

Using a marginal likelihood based on the ranks, with allowance for ties (*Jones and Whitehead (1979))*

the efficient score for θ is

$$Z = \frac{1}{n+1}\sum_{k=1}^{m} n_{kC}(L_{kT} - U_{kT})$$

and Fisher's information is

$$V = \frac{n_T n_C n}{3(n+1)^2}\left\{1 - \sum_{k=1}^{m}\left(\frac{n_k}{n}\right)^3\right\}$$

Application to Head Injury data

$$Z = \frac{1}{1108} \{73\,(0 - 118 - 66 - 10 - 92)$$
$$+ 55\,(219 - 66 - 10 - 92)$$
$$+ 79\,(219 + 118 - 10 - 92)$$
$$+ 37\,(219 + 118 + 66 - 92)$$
$$+ 358\,(219 + 118 + 66 + 10 - 0)\}$$

= 144.3

$$V = \frac{602 \times 505 \times 1107}{3 \times 1108^2} \left\{ 1 - \left\{\frac{292}{1107}\right\}^3 - \left\{\frac{173}{1107}\right\}^3 - \left\{\frac{145}{1107}\right\}^3 - \left\{\frac{47}{1107}\right\}^3 - \left\{\frac{450}{1107}\right\}^3 \right\}$$

= 91.38 (1 – 0.0917)

= 83.0

Hence

$$\frac{Z^2}{V} = 250.9$$

larger than any individual 2×2 table, and c.f. χ_1^2
very highly significant

Estimate of θ = $Z/V = 1.74$

between the values from the 2×2 tables

95% confidence interval for θ is

$$Z/V \pm 1.96/\sqrt{V} = (1.52, 1.96)$$

Note: The 2×2 tables contain 64%, 81%, 82% and 80% of total information respectively

The 2 × 2 table as a special case (m = 2)

	Control Group	Treated Group	Total
Success	s_C	s_T	s
Failure	f_C	f_T	f
Total	n_C	n_T	n

$$Z = \frac{1}{n+1}\left\{s_C\left(0-f_T\right)+f_C\left(s_T-0\right)\right\} = \frac{s_T f_C - s_C f_T}{n+1}$$

Note: n+1 instead of n in denominator

The 2 × 2 table as a special case (m = 2)

	Control Group	Treated Group	Total
Success	s_C	s_T	s
Failure	f_C	f_T	f
Total	n_C	n_T	n

$$V = \frac{n_C n_T}{3(n+1)^2}\left\{1 - \left(\frac{s}{n}\right)^3 - \left(\frac{f}{n}\right)^3\right\} = \frac{n_C n_T sf}{(n+1)^2 n}$$

Note: $(n+1)^2n$ instead of $(n)^2(n-1)$ in denominator

3.4 Relationship with the Mann-Whitney test

(Wilcoxon, 1945; Mann and Whitney, 1947)

Samples: $x_1, ..., x_a$ *(low values*

$y_1, ..., y_b$ *are good)*

Scores:

$$d_{ij} = \begin{cases} -1 \text{ if } x_i < y_j \\ 0 \text{ if } x_i = y_j \\ +1 \text{ if } x_i > y_j \end{cases}$$

Mann-Whitney statistic:

$$M = \sum_{i=1}^{a} \sum_{j=1}^{b} d_{ij} \qquad \text{var}\, M = ab(a+b+1)/3$$

(other variations exist)

Mann-Whitney test: $M^2 / \text{var}\, M \text{ c.f. } \chi_1^2$

Mann-Whitney test with ties

Values :	u_1	u_2	...	u_m	Total
x's	n_{1C}	n_{2C}		n_{mC}	n_C
y's	n_{1T}	n_{2T}		n_{mT}	n_T
Total	n_1	n_2		n_m	n

$$M = (n + 1)Z$$

Siegel (1957) gives variance of M with ties as

$$\operatorname{var} M = n_C n_T (n+1)/3 - n_C n_T \sum_{j=1}^{m} \left(n_j^3 - n_j\right) \Big/ \left\{3n(n-1)\right\}$$

$$= \frac{n_C n_T}{3n(n-1)} \left\{ n^3 - n - \sum_{j=1}^{m} n_j^3 + \sum_{j=1}^{m} n_j \right\}$$

$$= \frac{n_C n_T n^3}{3n(n-1)} \left\{ 1 - \sum_{j=1}^{m} \left(\frac{n_j}{n} \right)^3 \right\} \approx (n+1)^2 V$$

Thus, the score test under the proportional odds model is the Mann-Whitney test