

On likelihood methods for binary longitudinal data

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Binary longitudinal data

- ▶ A set of n individuals observed along time
- ▶ Response variable Y is binary (values: 0 and 1, say)
- ▶ Notation: response at time t from subject i is

$$y_{it} = \begin{cases} 0 \\ 1 \end{cases}$$

e.g. i th individual *profile* is $y_i = (1, 1, 0, 1, 0, 1)$

- ▶ Covariates, X_{it} , also recorded
- ▶ In general, want to relate X 's and Y



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- ▶ Partly a review and discussion
- ▶ Partly presentation of specific results
joint work with Helena Gonçalves (U. Algarve, Portugal)



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Outline

Brief review: approaches commonly in use

Transition models

Marginal models

Marginal likelihood-based models

Some new developments

Marginal models with MC2 dependence



Approaches: transition models

Transition models

- ▶ model transitions of an individual

$$\mathbb{P}\{Y_{it} = 1 | \text{past profile, covariates}\}$$

e. g.

$$\text{logit}(\mathbb{P}\{Y_{it} = 1 | y_{i,t-1}, \mathbf{x}_{it}\}) = \beta_0 + \beta_1 y_{i,t-1} + \beta_2 \mathbf{x}_{it}$$

- ▶ simple in formulation
- ▶ writing log-likelihood is immediate
using direct Markov chain connection
- ▶ OK if we want to model transitions
- ▶ but often we want to model marginal probability



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allowing for dependence within a given profile y_i

- ▶ dependence structure is ‘nuisance component’
- ▶ difficult to formulate fully-specified stochastic models with prescribed properties
- ▶ alternative route: do not attempt full stochastic specification



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Approaches: marginal models via GEE

Generalized estimating equations (GEE)

- ▶ no full stochastic specification
- ▶ model quantity of interest: $\mathbb{P}\{Y_{it} = 1 | \text{covariates}\}$
- ▶ requires specification of a ‘working correlation structure’, to accomodate correlation structure, compute std.errors ‘adjusted’ for presence of dependence
- ▶ Ok if we are only interested in the population behaviour
- ▶ cannot be used to tackle questions on individual profiles eg. $\mathbb{P}\{y_{i4} = 1 | \text{past} = (1, 0, 1), x_{it}\} = ?$



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 - (a) use standard likelihood-based inferences
 - (b) model population as well as individual behaviour
- ▶ aim at stochastic model for profile Y_j such that

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is represented by

$$\text{logit } \theta_{it} = \mathbf{x}^\top \beta,$$

or possibly other link function in place of logit

- ▶ from full stochastic specification, modelling of (serial) dependence must be allowed
- ▶ special interest for individual random effects



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'Mixed parameter' formulation

'Mixed parameter' formulation (Fitzmaurice & Laird, 1993)

- ▶ based on mixed mean and canonical association parameters in exponential families
- ▶ orthogonal regression parameters, β , and association parameters (α)
- ▶ various desirable features:
 - ▶ robustness to misspecification of time dependence
 - ▶ $\text{var}\{\hat{\beta}\}$ not influenced by knowledge of α , at least asymptotically
- ▶ some drawbacks:
 - ▶ association parameters are *conditional* log-odds ratios
 - ▶ distribution is not "reproducible", as profile length varies



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Markov chain re-parametrization

Markov chain re-parametrization (Azzalini, 1994)

- ▶ build 1st order Markov chain such that

$$\mathbb{P}\{Y_{it} = 1 | X_{it} = \mathbf{x}\} = \theta_{it} = \text{logit}^{-1}(\mathbf{x}^\top \beta)$$

and

$$OR(Y_{it}, Y_{i,t-1}) = \psi$$

by solving equation to get suitable transition matrix,
which depends on $(y_{i,t-1}, \theta_{it}, \theta_{i,t-1}, \psi)$

- ▶ desirable features:
 - ▶ likelihood expression is simple
 - ▶ parameter interpretation is transparent
 - ▶ orthogonal parameters, β and ψ
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Random effects

- ▶ random effects, in a simple case:

$$\text{logit}(\mathbb{P}\{Y_{it}|\mathbf{x}, \mathbf{b}_i\}) = \mathbf{x}^\top \boldsymbol{\beta} + \mathbf{b}_i$$

where $\mathbf{b}_i \sim N(0, \sigma^2)$

- ▶ problems:

- (a) computational, due to integration wrt dist'n of \mathbf{b}_i
- (b) interpretation of parameters, since

$$\mathbb{E}\left\{\frac{e^{\eta+b}}{1+e^{\eta+b}}\right\} \neq \frac{e^{\eta}}{1+e^{\eta}}$$

where $\eta = \mathbf{x}^\top \boldsymbol{\beta}$, hence meaning of $\boldsymbol{\beta}$ changes



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Marginalized random effects

Alternative formulation to random effects
(Heagerty, 1999; Heagerty & Zeger, 2000)

- ▶ similar logic of MC marginalisation is applied to random effects: find $\Delta = \Delta(\mathbf{x}, \sigma)$ such that

$$\text{logit}^{-1}(\eta) = \int_{-\infty}^{\infty} \text{logit}^{-1}(\Delta + \sigma \mathbf{z}) \phi(\mathbf{z}) d\mathbf{z}$$

- ▶ requires repeated solution of integral equation



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2nd order MC

- ▶ general idea: extend approach of Azzalini (1994) to 2nd order dependence
- ▶ specifically: formulate 2nd order MC such that

$$\mathbb{P}\{Y_t = 1 | X_t = x\} = \theta_t$$

(index i dropped) is given by

$$\text{logit } \theta_t = x^\top \beta$$

allowing dependence on (Y_{t-2}, Y_{t-1})

- ▶ in $2 \times 2 \times 2$ probability table of (Y_{t-2}, Y_{t-1}, Y_t) the above condition sets 3 probabilities, hence 4 parameters left
- ▶ parsimonious choice: use two parameters for modelling dependence, and add two constraints



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2nd order MC (cntd)

- ▶ Choice among possible parameters & constraints: impose

$$\begin{aligned} OR(Y_{t-1}, Y_{t-2}) &= \psi_1 = OR(Y_{t-1}, Y_t) \\ OR(Y_{t-2}, Y_t | Y_{t-1} = 0) &= \psi_2 = OR(Y_{t-2}, Y_t | Y_{t-1} = 1) \end{aligned}$$

- ▶ analogy with Gaussian AR(2) models, referred to OR in place of partial correlations
- ▶ technical problem: solve elements of MC transition matrix for given β, ψ_1, ψ_2
- ▶ very lengthy algebra but explicit solutions available



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Further work & related work

- ▶ obtain derivatives of logL to improve optimisation (even more messy algebra)
- ▶ allow for missing data (further complications. . .)
- ▶ random effects: possible but desirable to incorporate with Heagerty's (1999) approach
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Further work & related work

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