Does your PRO sum it all up?

Acaster Lloyd

Investigating the variability in item-specific PRO effects using random item slopes regression

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Motivating case

Research observations

Primary end-point is met; subsequent PRO end-points are not.

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Primary end-point is met; subsequent PRO end-points are not.

Lack of PRO ‘sensitivity’

Research observations

PROs are often used in conditions where they are not validated

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Diverse item content

Do all items that form PRO scores behave the same way?

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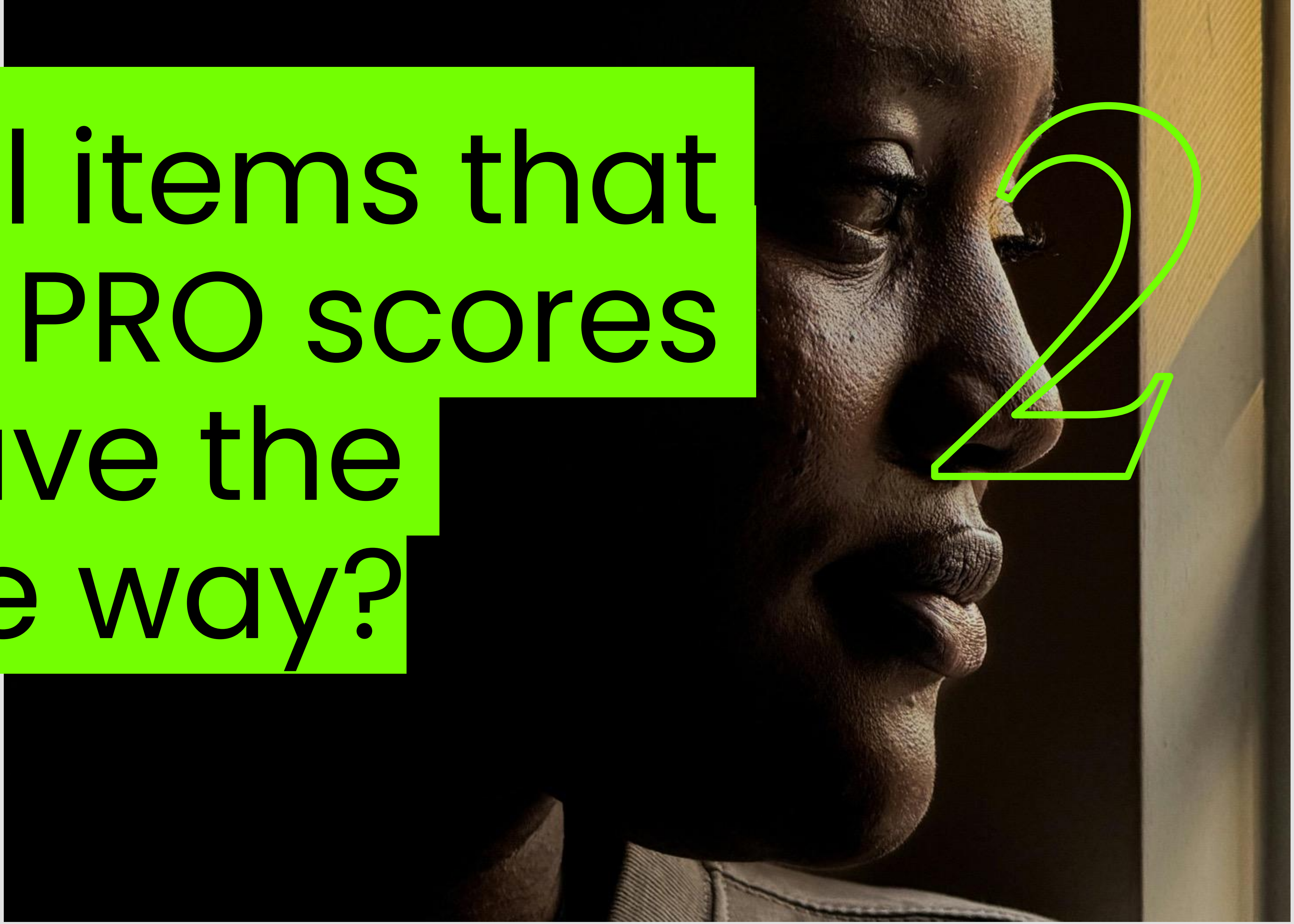

So, we assume they do.

Create an average or sum

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Run tests on this score

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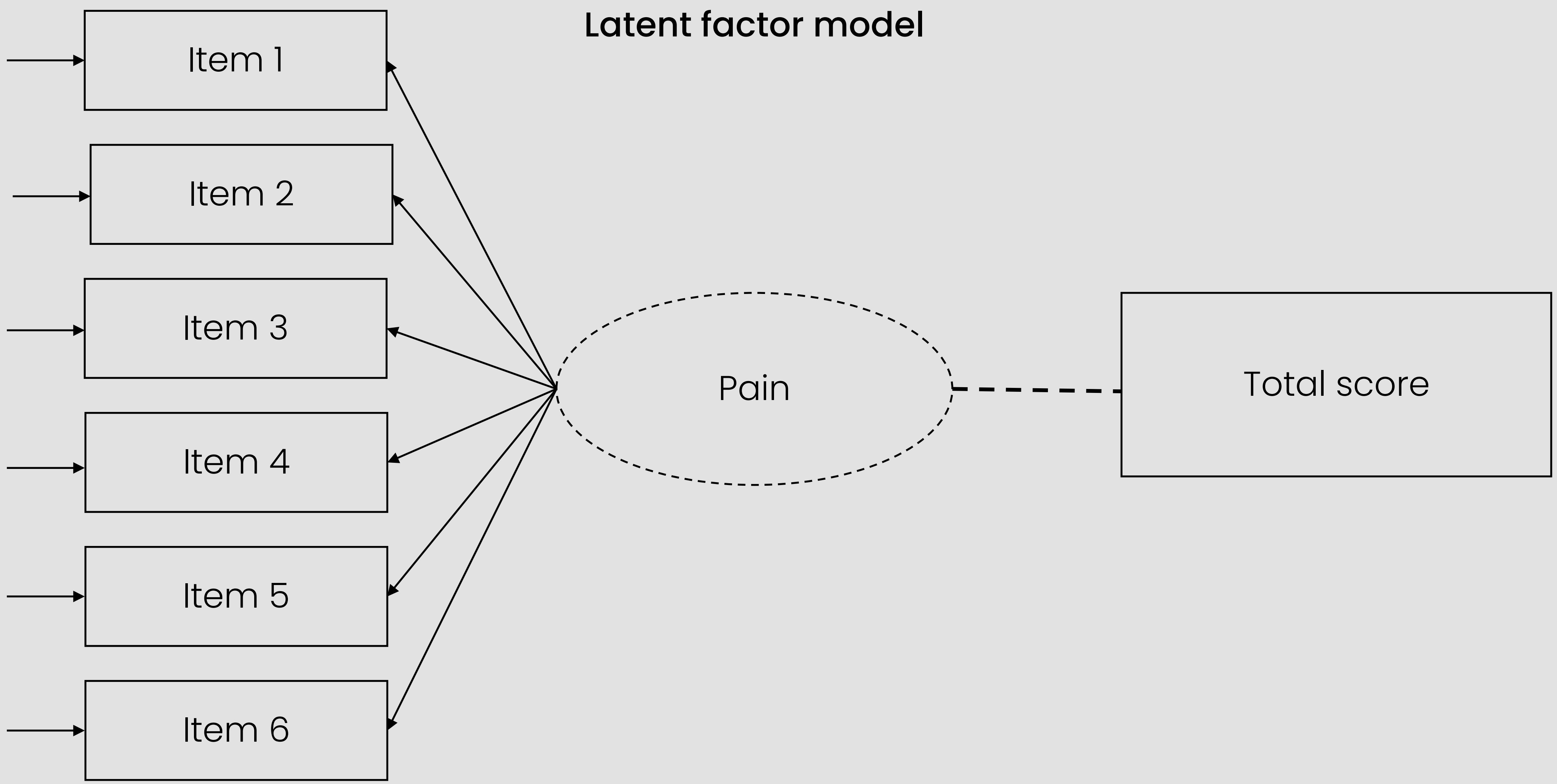
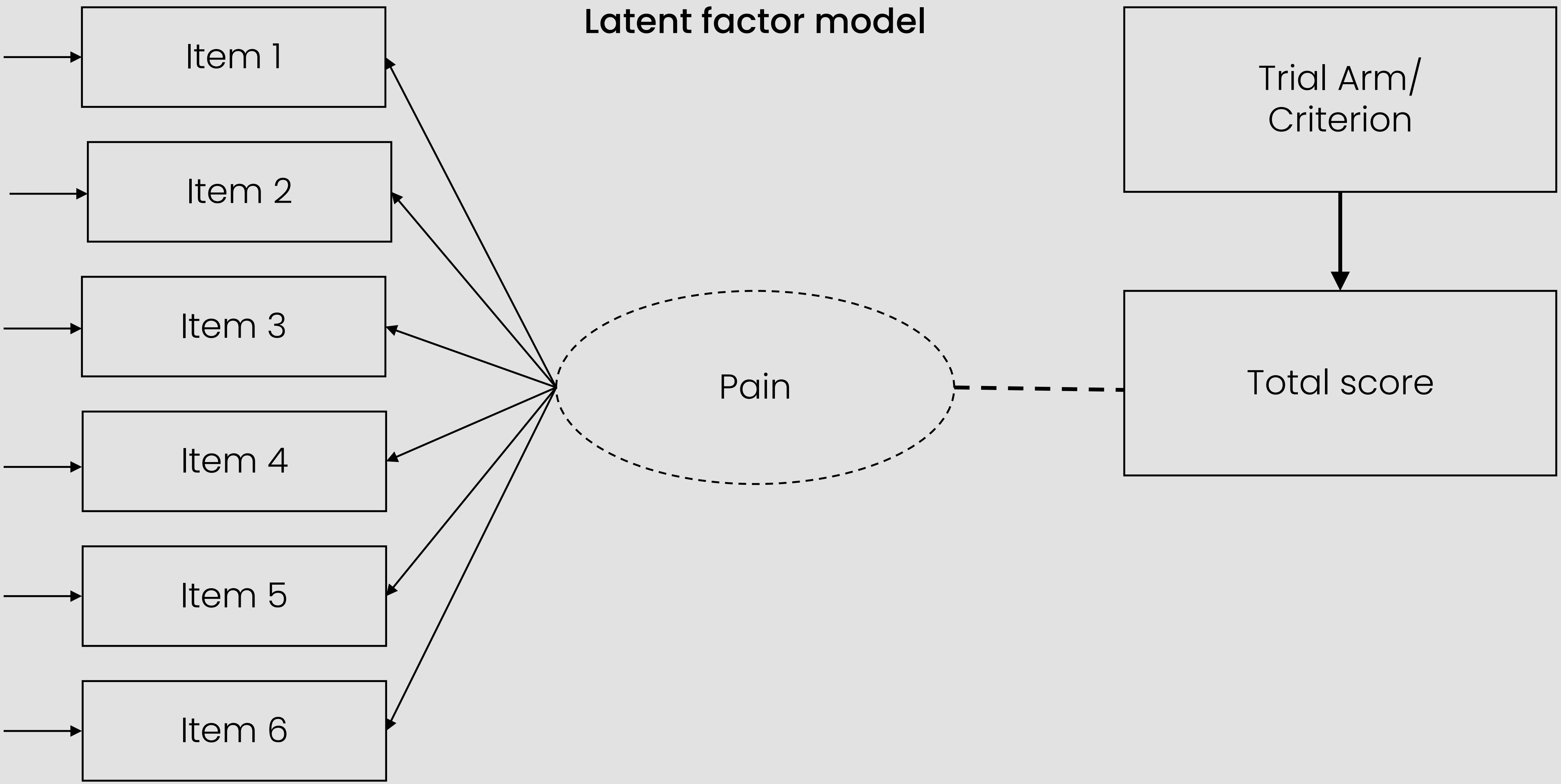
Draw a conclusion


Latent factor model
Item 1
Item 2
Item 3
Item 4
Item 5
Item 6
Pain
Total score


Latent factor model
Item 1
Item 2
Item 3
Item 4
Item 5
Item 6
Pain
Trial Arm/
Criterion
Total score

(Some) Potential issues in the typical approach

1. No test for sum score sufficiency

(Some) Potential issues in the typical approach

1. No test for sum score sufficiency
2. Lose nuance in understanding pattern of item effects

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Exploring variability in item level effects

Random item slope regression

Model item level effects via LMM

Donnellan, E., Usami, S., & Murayama K. (2025).
Psychological Methods 30, 4, 744-769

Simple “wide” data

ID	Pain Total Score	Criterion
ID101	3	12
ID102	3.2	9
ID103	1.1	11
ID104	5	8
ID105	4.8	14
ID106	2.1	10

Regression model for a total score

$$Pain_i = \beta_0 + \beta_1 Criterion_i + \varepsilon_i$$

Eq.1

$Pain_i$ = total pain score for participant *i*

β_0 = intercept

β_1 = fixed effect of criterion

ε_i = error

Flip our data long

ID	Pain Total Score	Criterion
ID101	3	12
ID102	3.2	9
ID103	1.1	11
ID104	5	8
ID105	4.8	14
ID106	2.1	10

ID	Item	Item Score	Criterion
ID101	item1	2	12
ID101	item2	3	12
...	...	...	...
ID101	item6	3	12
ID102	item1	1	9
...	...	...	...

Random item slope model

$$Item\ Score_{ij} = \beta_0 + \ v_{0i} + v_{0j} + (\beta_1 + v_{1j})Criterion_i + \ \varepsilon_{ij}$$

Eq.2

$Item\ Score_{ij}$ = response for participant *i* to pain item *j*

β_0 = model intercept

v_{0i} = random intercept for participant *i*

v_{0j} = random intercept for item *j*

Random item slope model

$$Item\ Score_{ij} = \beta_0 + \upsilon_{0i} + \upsilon_{0j} + (\beta_1 + \upsilon_{1j})Criterion_i + \varepsilon_{ij}$$

Eq.2

$\beta_1 Criterion_i$ = fixed effect of criterion

υ_{1j} = random slope for item *j*

Random item slope model

$$Item\ Score_{ij} = \beta_0 + \ v_{0i} + v_{0j} + (\beta_1 + v_{1j})Criterion_i + \ \varepsilon_{ij}$$

Eq.2

Random item slope model

$$Item\ Score_{ij} = \beta_0 + v_{0i} + v_{0j} + (\beta_1 + v_{1j})Criterion_i + \varepsilon_{ij}$$

Eq.2

v_{1j} Captures the variability in the effect of the criterion on item responses

Random item slope model

$$Item\ Score_{ij} = \beta_0 + v_{0i} + v_{0j} + (\beta_1 + v_{1j})Criterion_i + \varepsilon_{ij}$$

Eq.2

Estimate of β_1 gives the average effect we wanted from Eq.1

Random item slope model

$$Item\ Score_{ij} = \beta_0 + \ v_{0i} + v_{0j} + (\beta_1 + v_{1j})Criterion_i + \ \varepsilon_{ij}$$

Eq.2

Estimate of β_1 gives the average effect we wanted from Eq.1

But…when v_{1j} is not 0, provides an unbiased estimate of the SE

Very brief example

Mini "simulation"

N = 300

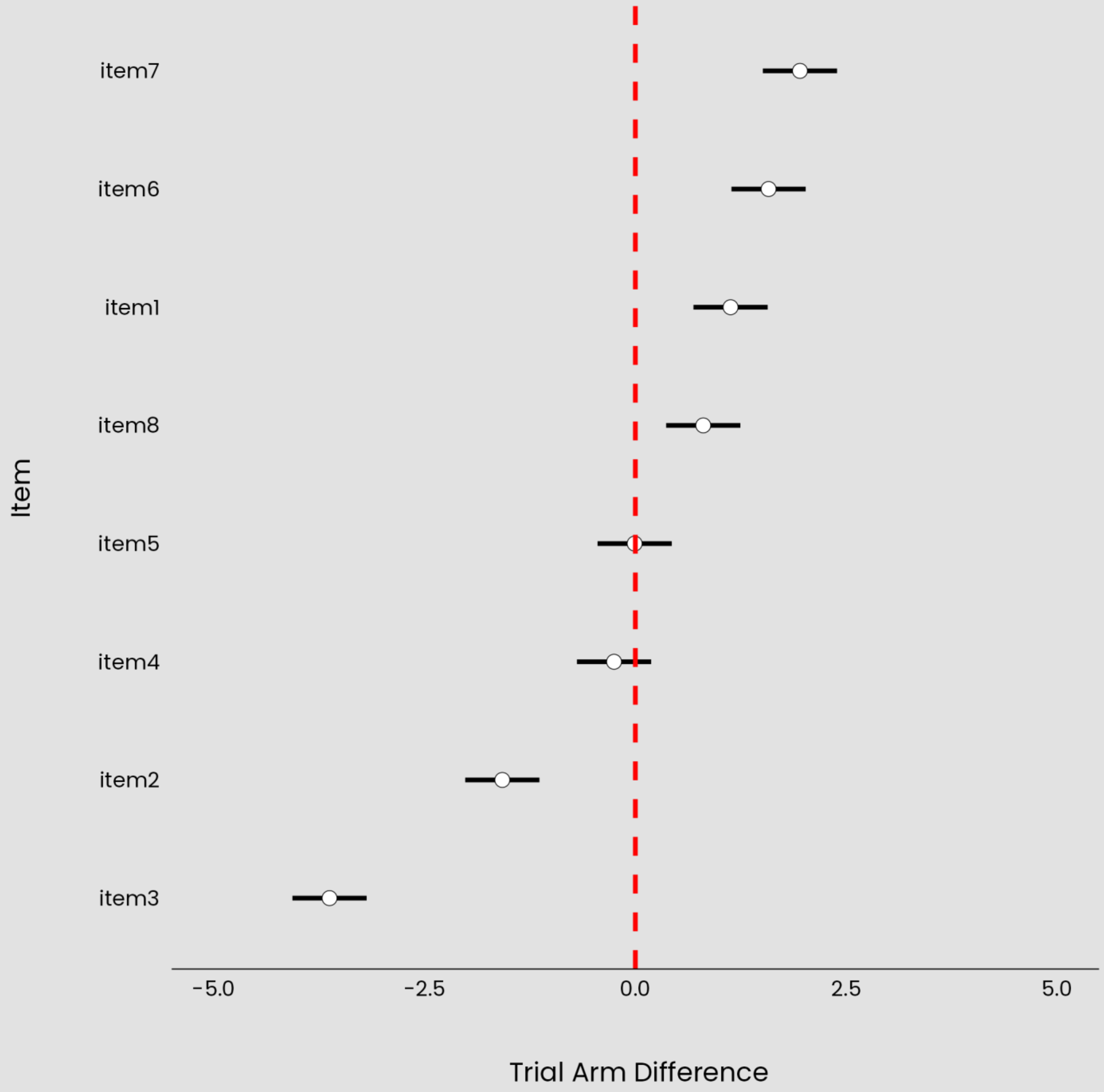
Fixed effect of arm = 1

Varied the size of the random slope by item

All other parameters held constant

Variability in slope	**Total score LM (Fixed Est, SE)**	**Random Slope (Fixed Est, SE)**
0.5	0.83, 0.03	0.83, 0.15
1.0	0.63, 0.03	0.63, 0.40
1.5	1.30, 0.03	1.30, 0.66

Visualising item level variability



Can we extend the model?

Any LMM model set up

Including explanatory variables for the random slopes

Potential applications

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Where might the approach be most valuable?

1. Exploratory item analysis
2. Measure development and validity assessment

Quick recap

Efficient exploration of item specific effects

All the flexibility of linear mixed models

Applications in both post-hoc analysis and PRO validation

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