Machine-Learning Methods to Investigate Heterogeneity in Longitudinal Patient-Reported Outcome Measures

University of Manitoba

The VADA Program
Visual and Automated Disease Analytics Graduate Training Program

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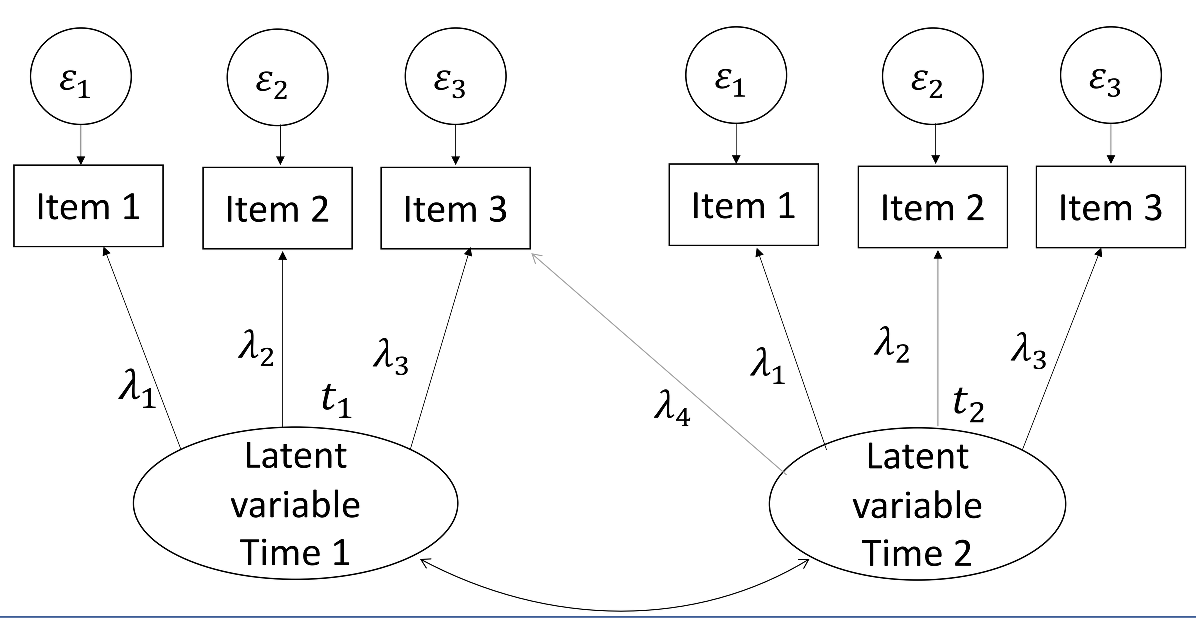
INTRODUCTION

- **Patient-reported outcome measures (PROMs)**
  - Appraisals from patients about their well-being and quality of life
  - Are comprised of multi-item self-report scales that capture perceptions about physical, mental, and social health
  - Provide the patient's perspective on the effectiveness of healthcare interventions, such as surgeries or health programs
  - Are sensitive to differences in interpretation by sub-groups within the population
- **What is Response Shift in PROMs?**
  - Patients may change their interpretation of the items that comprise a multi-item scale over time (i.e., at multiple measurement occasions)
  - Results in inconsistencies in measurement properties of multi-item scales collected over time. Types of response shift: recalibration, reprioritization, and reconceptualization



Note: Item 1, 2, and 3: observed variables, $\varepsilon_1, \varepsilon_2$, and ε_3: error terms, λ_1, λ_2, λ_3, and λ_4: factor loadings

- **Recalibration**: Change in measurement structure leads to changes in error variances over time
- **Reprioritization**: Change in the relative importance of items and latent variables contribute to change in factor loadings over time
- **Reconceptualization**: Change in the way the latent variable is conceptualized leads to changes in factor loading patterns over time

Figure 1: Latent variable model at two measurement occasions

- Observed variables may be responsible for variation in response shift and result in response shift patterns.

- **Item Response Theory Models for Response Shift**
  - Item Response Theory (IRT) models have been used to identify individual PROM scale items that are sensitive to response shift
  - Conventional IRT models assume homogeneity in parameter estimates over time may result in imprecise response shift, if heterogeneity exists
  - Unsupervised machine-learning methods can aid in identifying clusters of individuals with similar response shift patterns

PURPOSE & OBJECTIVES

Purpose: To develop new methods to detect heterogeneity (i.e., variation) in response shift in longitudinal PROMs data

Objectives:

- Develop a longitudinal IRT model that uses unsupervised machine-learning techniques to detect response shift in PROMs data
- Compare this new model with existing statistical models (i.e., conventional IRT model) to detect response shift
- Apply this newly-developed model to real-world clinical data about PROMs for patients having joint replacement surgery

Research Questions

- Do unsupervised and conventional longitudinal IRT models differ in their sensitivity to detect variation in response shift in PROM item responses?
- Are longitudinal unsupervised and conventional IRT equally sensitive to detect different types of response shift in PROMs item responses?

METHODS

Statistical Models

- The implementation of the **conventional IRT models** will follow the steps below.
  - Establish the measurement model for the latent construct (e.g., mental health, physical health)
  - Test for an overall response shift effect
  - Test for response shift on each PROM item
  - Fit the final response shift model and use it to estimate change in the latent variable scores for each individual
- The implementation of the **unsupervised machine-learning IRT model** will follow the steps in **Figure 2**.

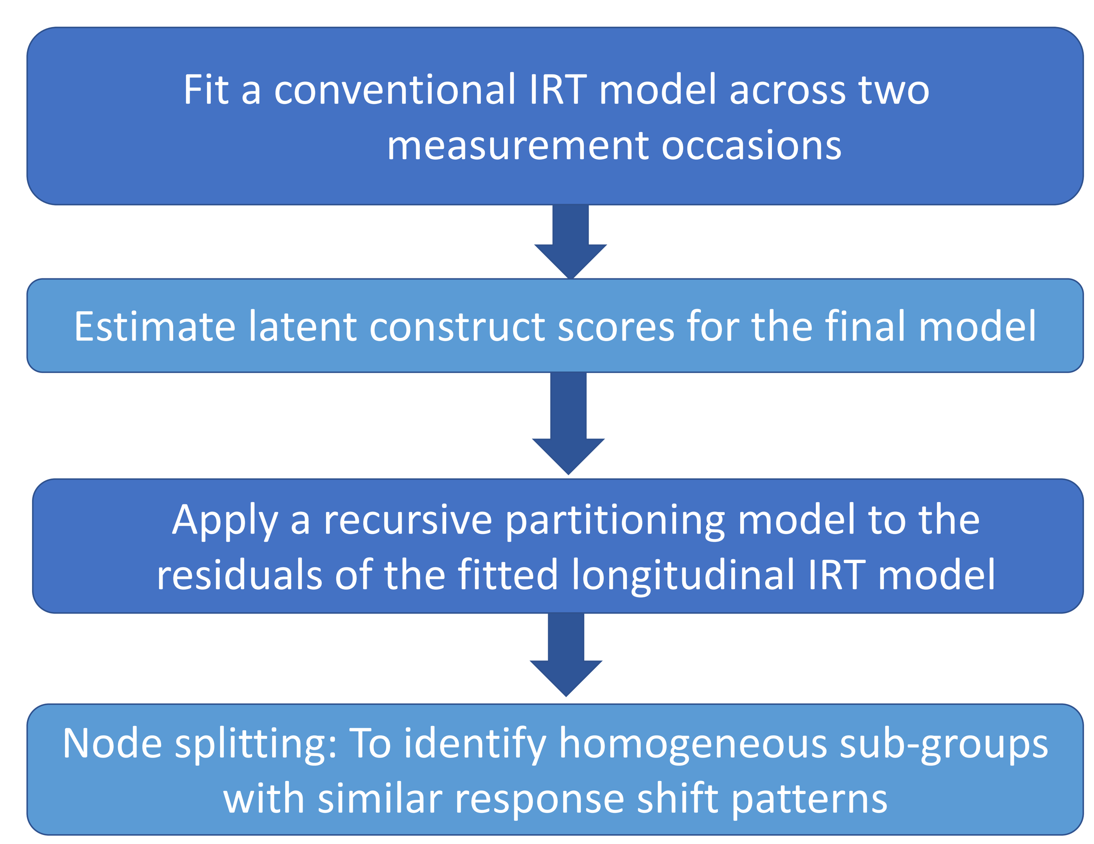


Figure 2: Implementation of unsupervised IRT model

Computer Simulation Studies

- Models will be compared using computer simulations to detect response shift.
- **Simulation study characteristics:**
  - ***Research design characteristics***: total sample size
  - ***Response characteristics***: number of PROMs items measured using an ordinal scale, number of item response categories, type of response shift effect
  - ***Covariate effects:*** number of covariates associated with response shift (i.e., number of clusters with similar response shift patterns)
  - ***Covariance/correlation***: magnitude of correlation among the measurement occasions, variance of the covariates
  - ***Effect size***: magnitude of the true difference between measurement occasions; response shift effect size.

We will then apply two models to real-world data from WRHA

Clinical Data

- Winnipeg Regional Health Authority (WRHA) Joint Replacement Registry
- Covariates: socio-demographic and clinical characteristics
- Both general-purpose and condition-specific PROMs are captured in the data (Table 1).
- Longitudinal Data: PROMs are collected one month prior to surgery and one year following surgery

General-purpose PROMs	Condition-specific PROMs
Short Form Health Survey (SF-12): used to assess both mental and physical health	**Oxford Hip Score:** assess function and pain before and after hip replacement surgery
	Oxford Knee Score: assess function and pain before and after knee replacement surgery

SIGNIFICANCE

- WRHA Joint Replacement Registry contains a rich set of covariates
- Contribute to the development of valid and sensitive PROMs methods
- Help to advance the use of unsupervised machine-learning methods for PROMs data
- Valid analytic techniques will contribute to better interpretation of the patient's perspective

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