A Bayesian Data Analysis In a Small n Sequential Multiple Assignment Randomized Trial (snSMART)
2018 Joint Statistical Meetings

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Study Design

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Baseline 6 months 12 months
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```
R 1:1:1

A  
Response?  Yes  A
No  B

R 1:1
B  
Response?  Yes  B
No  A

R 1:1
C  
Response?  Yes  C
No  A

R 1:1
A  
B

C

```

Boxian Wei (University of Michigan)  Bayesian snSMART  July 30, 2018
Notation

- **Outcome** - $Y_{ijk}$: a binary response for $i^{th}$ patient at the $j^{th}$ stage receiving treatment $k$ ($k = A, B, C$).

- **Primary interest** - $\pi_k$: efficacy of individual treatment $k$, which is measured by the response rate associated with the treatment $k$.

- **Linkage parameter** - $\beta_1k, \beta_0k$: is used to model the second-stage response conditionally on the first-stage response.
  - $\beta_1k \pi_k$: second stage response rate on treatment $k$ for first stage responders.
  - $\beta_0k \pi_k'$: second stage response rate on treatment $k'$ for first stage non-responders.
Bayesian Joint Stage Model (BJSM)

- For $i^{th}$ patient at the 1$^{st}$ stage receiving treatment $k$.
  \[ Y_{i1k} | \pi_k \sim Bernoulli(\pi_k) \]

- For $i^{th}$ patient at the 2$^{nd}$ stage receiving treatment $k$ or $k'$.
  \[ Y_{i2k(k')} \mid Y_{i1k}, \pi_k \sim Bernoulli( (\beta_1 \pi_k)^{Y_{i1k}} (\beta_0 \pi_{k'})^{1-Y_{i1k}} ) \]

- Prior settings
  - $\pi_k \sim Beta(0.4, 1.6)$, $\beta_1 \sim Pareto(3, 1)$, $\beta_0 \sim Beta(1, 1)$
Comparator Models

- Log-Poisson Joint Stage Model (LPJSM)
- Bayesian Joint Stage Model with Multiple Linkage Parameters (BJSMM)
- Bayesian First Stage Model (BFSM)
Simulation Settings

- We look at the following statistics of the estimators when we compare our methods with others.
  - Bias, Root mean-square error (rMSE), Length of 95% credible/confidence (CI), Coverage rate of 95% CI

- Simulation Scenarios

**Table 1**: Simulation scenarios for snSMART with 30 patients per arm.

<table>
<thead>
<tr>
<th>Scenarios</th>
<th>$\pi_A$</th>
<th>$\pi_B$</th>
<th>$\pi_C$</th>
<th>$\beta_{0A}$</th>
<th>$\beta_{0B}$</th>
<th>$\beta_{0C}$</th>
<th>$\beta_{1A}$</th>
<th>$\beta_{1B}$</th>
<th>$\beta_{1C}$</th>
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<td>0.3</td>
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<td>0.6</td>
<td>0.6</td>
<td>0.6</td>
<td>1.5</td>
<td>1.5</td>
<td>1.5</td>
</tr>
<tr>
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<td>0.2</td>
<td>0.3</td>
<td>0.4</td>
<td>0.9</td>
<td>0.6</td>
<td>0.3</td>
<td>1.2</td>
<td>1.5</td>
<td>1.8</td>
</tr>
</tbody>
</table>
Summary of Simulation Results

- In both scenarios, the BJSM and BJSMM perform well across all measures.

- In most other scenarios, the BJSM and BJSMM outperform the other methods.
Future Work

- Establish sample size calculations based on the analysis of snSMARTs using the Bayesian joint stage model.
- Develop an easy-to-use applet of sample size calculation for user specified study design.
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Thank you!

Q & A

Please follow up
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