Bayesian Data Envelopment Analysis (DEA) for Assessing Drug Benefit-Risk

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BACKGROUND

- Commonly used benefit-risk (BR) methods[1-7] do not allow combining all benefit and risk profiles into a straightforward quantitative comparison of treatment that recognizes the uncertainty in estimation.
- Although Waddingham et al.[8] proposed a Bayesian MCDA approach that could propagate uncertainty due to sampling error and make statistical inference based on the posterior distribution of BR scores, their approach requires elicitation of weights that could lead to biased results if inaccurate.

METHODS

- DEA is a nonparametric mathematical programming method for evaluating the relative efficiency of decision-making units (DMUs) with multiple criteria that are termed as outputs and inputs. It performs measurement on a linear combination of outputs over a linear combination of inputs, explores all weight combinations, estimates the maximum possible outputs for a given number of inputs, and provides relative efficiency scores as results.
- In drug BR assessment, outputs are drug benefits and inputs are risks of drugs.
- We propose a Bayesian approach to propagate uncertainty in the measurement of outcomes and enable statistical inference in the BR assessment framework.

DATA

- We investigate and compare model performance via a simulation study.

RESULTS

- Bayesian DEA is a promising alternative method in drug BR assessment.

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Bayesian Data Envelopment Analysis (DEA) Method Shows Promising Result in **Drug Benefit-Risk Assessment**

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Flowchart of the Bayesian DEA approach

1. Collect summary statistics of endpoint variables (benefit outcome or risk outcome) from clinical trials.
2. Fit Bayesian model and get the posterior draws of response rates for each endpoint variable.
3. Fit the posterior response rates into DEA inequalities and obtain DEA efficiency score posterior distributions for each drug.
4. Perform tests based on the DEA efficiency score posterior distributions and compare drug efficiency.

Bayesian Model

$x_{j,k}$: number of subjects out of $n_{j,k}$ subjects treated with drug $j = 1, \ldots, J$ that experience outcome $k = 1, \ldots, K$

$\pi_{j,k}$: proportion of subjects experiencing outcome k for drug j

$$x_{j,k} \mid \pi_{j,k} \sim Bin(n_{j,k}, \pi_{j,k})$$

Uninformative prior on log-odds of the response rate:

$$logit(\pi_{j,k}) = \mu_{j,k}$$
$$\mu_{j,k} \sim N(0,10)$$

Simulation Design

- Five potential drug treatments, $J = 5$
- Impact of number of outcome measures was investigated:
  $K = (2, 4, 5, 10)$
- Null scenario assumes $\pi_{j,k} = 0.25$ for all j, k
- Alternative scenarios assume 1 or more winners
- Winner(s) defined by absolute differences in response rates for 1 or more outcomes, e.g.,:
  effect size = $\pi_{j,k} - \pi^{'}_{j,k} = (0.05, 0.1)$
- Impact of sample size was investigated:
  $n_{j,k} = (100, 300, 500, 700, 900, 1100)$

Testing for DMUs with Superior Efficiency

- Do credible intervals (CI) of efficiency scores separate?
  - ➢ Conclude there are DMUs with superior efficiency

- *Type 1 Error Calibration*

	CI length=60	CI length =50	CI length =40	CI length =30	CI length =20	#simulations
2 DEA variables	**0.039**	0.116	0.278	0.534	0.809	18000
4 DEA variables	0.014	**0.043**	0.1	0.197	0.329	6000
5 DEA variables	0.006	0.018	**0.04**	0.078	0.135	18000
10 DEA variables	0	0	0	0	0	6000

- *Power*