

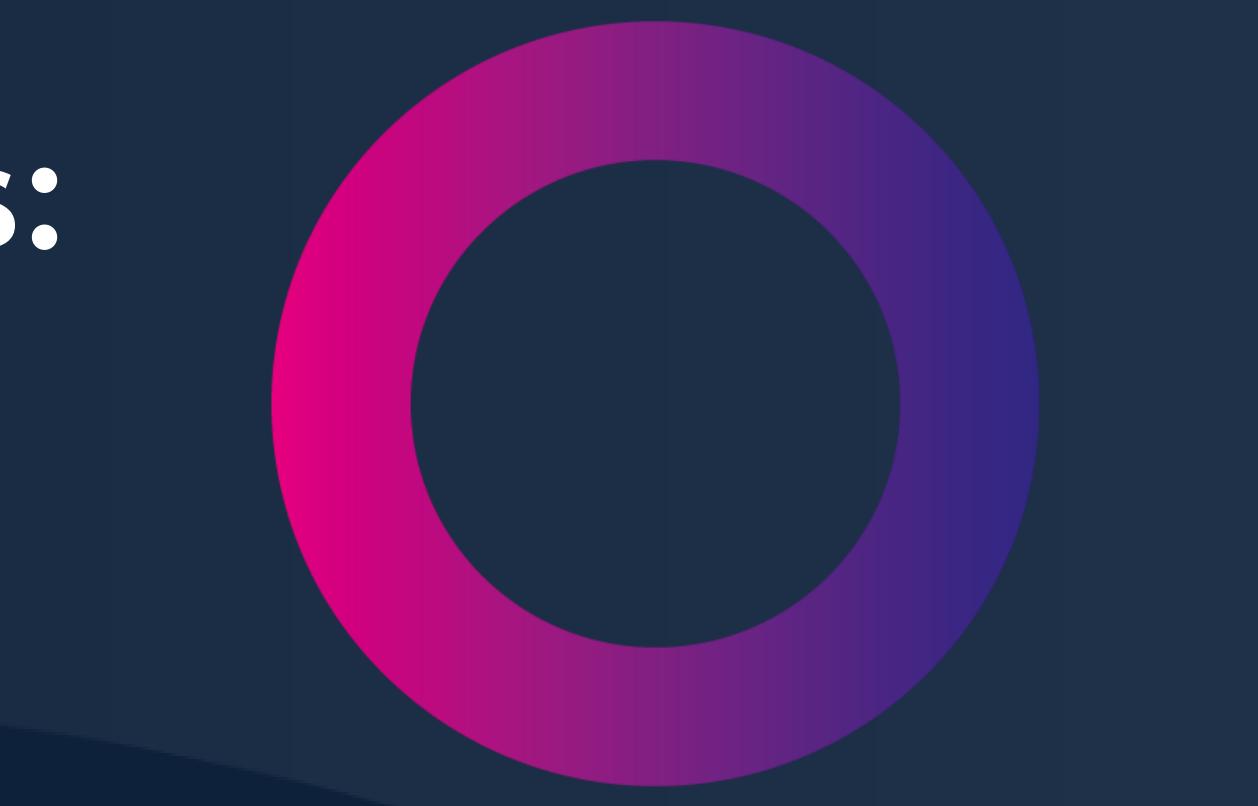
# Comparing Choice Share Prediction Methods for Discrete Choice Experiments: A Case Study With Hypomethylating Agents in Myelodysplastic Syndromes

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## INTRODUCTION

- Discrete choice experiments (DCEs) can be used to produce choice share predictions, translating patient preferences to product demand.
- In the healthcare literature, choice share predictions are conventionally calculated using the ‘share of preference’ method. An alternative, but debated, method is the first-choice method. There has been little investigation on differences in predictions between the methods in healthcare.
- A case study was conducted with myelodysplastic syndromes (MDS) patients to investigate their treatment preferences for hypomethylating agents (HMA) and compare various choice share prediction methods.
  - MDS is a disease of the hematopoietic stem cells and can result in an increased risk of developing acute myeloid leukemia (AML).<sup>1</sup>
  - HMA is considered the standard of care for MDS patients who are ineligible for haemopoietic stem cell transplant (HSCT).<sup>2</sup>

## OBJECTIVES

- To compare accuracy of choice share predictions using different methods with an in-survey discrete-choice experiment (DCE) holdout task consisting of real-life HMA choices.

## METHODS

### Study Design and Population

- A cross-sectional online DCE survey was conducted to elicit preferences of MDS patients (and caregivers serving as patient proxies), comparing benefits, risks, and administration burden of HMAs.
- Respondents were recruited via the networks of patient organizations in the US and Canada (i.e., MDS Foundation, the Aplastic Anemia and MDS International Foundation, and the Aplastic Anemia and Myelodysplasia Association of Canada).
- Respondents were eligible for the study if they were 1) an MDS patient or a caregiver of a patient with MDS, responding as a proxy for the patient, 2) 18 years of age or older, 3) living in the US or Canada and 4) able to read and understand English.
- The study protocol was submitted to the Advarra Institutional Review Board and determined to be exempt from IRB oversight.

### Study Survey

- Six treatment attributes of three categories were included: (1) efficacy (chance of developing AML), (2) risk (fatigue), and (3) administration (mode of administration, frequency of administration, number of visits, duration of visit) (Table 1).
- Attributes and levels were selected to reflect current and upcoming HMA treatments and were based on a targeted literature review and qualitative interviews with clinicians experienced in treating MDS patients (n=3), patients (n=10), and caregivers (n=6).

Table 1. Treatment Attributes and Levels

Attributes/Labels	Short Description	Attribute Levels
Mode of Administration	This describes how you receive medication	Oral pill, SC injection, IV infusion
Frequency of Administration	How frequently you take the medication	5 days/7 days/14 days straight, repeat every month
Number of Visits	Number of visits to the doctor's office to receive medication and/or medical consultation/advice	1 visit/5 visits/7 visits every month
Duration of Visit	This describes how you receive your medication, the time you need to travel to and from, and stay at, the doctor's office and the consultation/advice you receive by the doctor.	1.5 hours, 2 hours, 3 hours, 4 hours
Chance of Developing AML	The chance you may develop AML within one year	30%, 35%, 40%, 50%
Level of Fatigue	This describes the level of fatigue associated with disease	No fatigue, mild fatigue, moderate fatigue, extreme fatigue

- Each respondent was shown 14 choice tasks (D-efficient design) with three options: two treatments and an opt-out.
- Respondents were presented a fixed holdout task comprised of four available marketed real-life HMA products (an oral pill, two infusions, and one injection) and an opt-out (Figure 1).

Figure 1. Example of the holdout task for real-life HMA products

	Medication A	Medication B	Medication C	Medication D	No Medication
Mode of Administration	IV infusion	SC injection	IV infusion	Oral Pill	None
Frequency of Administration	7 days	5 days	5 days	5 days	None
Number of Visits	7 visits	5 visits	5 visits	1 visit	1 visit
Duration of Each Visit	4 hours	1.5 hours	4 hours	1.5 hours	1.5 hours
Chance of Developing AML	30%	30%	30%	30%	50%
Level of Fatigue	Mild fatigue	Mild fatigue	Mild fatigue	Mild fatigue	Severe fatigue
The best choice is:					
The worst choice is:					

## METHODS (CONT.)

### Analysis

- The DCE responses were analyzed with a multinomial logit (MNL) and a random parameters logit (also called a “mixed logit”) model (MXL). The MXL model assumes that the probability of choosing a treatment profile is a function of both the attributes levels and a random error that adjusts for individual-specific variations in preferences.
- Predictions were made for the holdout task using three methods of estimating choice share predictions: (1) first-choice method per MXL model draw, (2) share-of-preference MXL (incorporating heterogeneity), and (3) share-of-preference MNL.
- Respondents’ choices in the holdout task were compared to the three choice share predictions and the mean absolute error (MAE) was calculated for each prediction.

## RESULTS

- Participant characteristics are reported in Table 2. More than half (56%) of MDS patients were currently on treatment; 24% had previously received treatment but discontinued.

Table 2. Participant characteristics

Variable	Statistic or Category	All Respondents (N = 184)
Participant type, N (%)	Patient	158 (85.9%)
	Caregiver/Proxy	26 (14.1%)
Age (Years) <sup>a</sup>	Mean (SD)	67.2 (10.0)
	Median (Q1 to Q3)	69.0 (62.0 to 73.0)
	Range	27.0 to 91.0
Sex, N (%)	Male	93 (50.5%)
Race, N (%)	African American or Black	5 (2.7%)
	Asian	5 (2.7%)
	White	168 (91.3%)
	Other	6 (3.3%)
Geographic location <sup>a</sup>	United States	158 (87.8%)
	Canada	22 (12.2%)
IPSS-R score, N (%)	1.5 or lower (very low)	26 (14.1%)
	2-3 (low)	38 (20.7%)
	3.5-4.5 (intermediate)	35 (19.0%)
	5-6 (high)	14 (7.6%)
	6.5 or higher (very high)	8 (4.3%)
	Unknown	63 (34.2%)

IPSS-R = Revised International Prognostic Scoring System (IPSS-R); SD = standard deviation

<sup>a</sup> Age calculated for n=183 participants, less missing data from one participant.

<sup>b</sup> Geographic location calculated for n=180 participants, less missing data from four participants.

Note: Due to rounding, percentages may not add up to 100%.

- The MXL model had the best fit based on AIC and BIC.

- The first-choice MXL prediction was most accurate, that is, showing the lowest MAE (1.3%, see Table 3).
- The predictions were less accurate for the share-of-preference MXL (MAE of 9.5%) and the MNL (MAE of 13.4%).

Table 3. Actual Responses, Choice Predictions, and MAE Calculations

Treatment Options	Actual Responses	MXL-First Choice	MXL- Share of Preferences	MNL
Choice Predictions				
Oral Pill	77%	77%	59%	45%
SC Injections	15%	11%	9%	13%
IV Infusions, 7 Visits	3%	4%	20%	25%
IV Infusions, 5 Visits	5%	6%	11%	16%
No Treatment	1%	2%	2%	2%
MAE Calculations				
Oral Pill	0%	18%	32%	
SC Injections	3%	6%	2%	
IV Infusions, 7 Visits	1%	16%	22%	
IV Infusions, 5 Visits	1%	6%	11%	
No Treatment	2%	2%	1%	
Average MAE	1.3%	9.5%	13.4%	

## CONCLUSIONS

- The first-share predictions closely resembled the holdout task choices, where the conventional share-of-preference prediction method did not.
- Research is required to understand whether this result is generalizable (e.g., when actual choices are less extreme) and to provide researchers with guidance on the prediction method.

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## DISCLOSURES

JHT, JS, and SJ are employees of OPEN Health, and MK, MB and EZ are former employees of OPEN Health. In addition, MB owns shares of OPEN Health. OPEN Health is a research consulting company that received funding from Taiho Oncology to conduct this study.