The utilization of natural language processing (NLP) to gauge healthcare decision-maker perceptions of cell and gene therapies (CGTs)

Xcenda, L.L.C., Carrollton, TX, USA

Background

- Cell and gene therapies (CGTs) represent a rapidly evolving market with the potential to provide significant clinical and economic benefits to patients, caregivers, and society at large.
- However, high costs and clinical uncertainties have created varied healthcare decision-maker (HCDM) perceptions and barriers to coverage, reimbursement, and patient access.
- Methods to readily assess HCDM perceptions of CGTs are needed to facilitate relevant and timely discussions that enable biopharma companies to tailor evidence generation and market access strategies to meet the needs of HCDMs.
- Artificial intelligence (AI), such as natural language processing (NLP), is increasingly being utilized in healthcare to improve outcomes and care delivery.^{1,2} This includes evaluating large, unstructured datasets to inform real-time decision-making.
- FormularyDecisions[®] is an online platform connecting biopharma companies to their HCDM customers. HCDMs have access to a variety of resources to inform formulary decision making and the ability to provide valuable insights to biopharma companies through product-specific surveys.
- Sentiment analysis is an NLP technique that can be used to automatically determine whether qualitative survey data is negative, neutral, positive, or mixed.²

Objective

• To assess qualitative HCDM survey responses regarding CGTs using a Microsoft (MS) NLP sentiment analysis.

Methods

- Open-ended survey responses and accompanying numerical ratings from November 15, 2018 to May 31, 2022 were collected from FormularyDecisions to gather insights on the clinical efficacy and economic value of 3 FDA-approved CGTs with active subscriptions.
- Survey respondents included HCDMs from managed care organizations (MCOs), pharmacy benefit managers (PBMs), academic institutions, provider organizations, consultant agencies, government, and other organizations who were verified to have a role in the formulary decision-making process.
- The AI Builder capability from the MS Power Platform was used to generate an NLP sentiment analysis and automatically process the open-ended survey responses
- Responses were categorized into the following sentiment valence types: positive, negative, neutral, mixed.
- Descriptive statistics were used to evaluate categorical trends. Results are reported in aggregate.
- To validate the Al's categorization of sentiments into valence categories, numerical ratings for clinical efficacy and economic value by sentiment valence type were assessed through a one-way analysis of variance (ANOVA).



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Results

Respondent demographics

Figure 1: Count of survey responses by organization type^a



^aIncludes respondents from organizations such as academia, government, and consultants. Key: MCO – managed care organization; PBM – pharmacy benefit manager.

- There was no substantial difference among positive, neutral, or negative sentiment across the 3 products. The distribution of overall sentiment was slightly more negative (40.3%) and neutral (28.6%) (**Figure 2**).
- Sentiment for clinical efficacy was 37.1% neutral (Figure 3), while 55.5% of responses for economic value were classified as negative (**Figure 4**).

Figure 2: Distribution of overall sentiments^a

40.3%



N=563. Note: The sum of percentages may not equal 100% due to rounding. ^aFigure reflects aggregate sentiment data for clinical efficacy and economic value. Q: What is your current overall perception of the [clinical efficacy/economic value] of each of the following products (based on the assumption of price parity with the primary comparator if a product was pre-approval)? Why did you rate [clinical efficacy/economic value] as you did?

- (44.2%) (**Figure 5**).
- for economic value across all organization types.
- PBM organizations (33.3%) (**Figure 6**).
- largely negative sentiment of at least 40.0% (**Figure 7**).
- was a significant difference for economic value ratings.
- compared to negative types (P<0.05; P<0.001).

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Breyanne Bannister, PharmD; Joseph Washington, PharmD, MPH; Claire Gorey, MA; Andrew Gaiser, PharmD, MBA, MS; Justin Merritt; Melissa McCart, PharmD, MS

• There were 563 total survey responses; 280 for clinical efficacy and 283 for economic value. Respondents were primarily from managed care (247; 43.9%), provider (149; 26.4%), and PBM (120; 21.3%) organizations (**Figure 1**).

• Overall sentiment was most positive across MCOs (28.7%) and provider (22.8%) organizations, and most negative across other (50.5%) and PBM organizations

In general, sentiment was more positive for clinical efficacy and more negative

– For clinical efficacy, positive sentiment was highest among MCOs (38.5%) and PBMs (28.3%), while negative sentiment was highest among other (38.6%) and

- Positive sentiment for economic value was highest among provider organizations (23.0%) followed by MCOs (19.2%). All organization types had a

 One-way ANOVAs suggested there was no statistically significant difference in numerical clinical efficacy ratings across sentiment valence types; however, there

 Post hoc analyses were conducted to further evaluate the significant difference in economic value ratings. Pairwise comparisons suggested that economic value ratings were significantly higher for neutral and positive sentiment valence types

Figure 3: Distribution of sentiment for clinical efficacy



n=280. Note: The sum of percentages may not equal 100% due to rounding.

^aOpen-ended responses have been blinded to product mentions or product-specific characteristics. Other organization types include respondents from organizations such as academia, government, and consultants.

Q: What is your current overall perception of the clinical efficacy of each of the following products (based on the assumption of price parity with the primary comparator if a product was pre-approval)? Why did you rate clinical efficacy as you did? Key: MCO – managed care organization; PBM – pharmacy benefit manager.

Figure 4: Distribution of sentiment for economic value

Sample open-ended responses by sentiment type^a

Vegative		"The cost is a significant challenge for payers. Not just the dollar amount, bu where the payer assumes all financial risk without guarantee of future premi
55.5%		"All are expensive, but [Product X] is the most costly for us." – Other organize
	Neutral	"As I think the clinical aspects of the products are similar and the cost is the equal." – Provider organization
	20.1%	"Specific economic data is not readily available; however, the expectation is price] per patient." – PBM organization
		"While the data shows pretty similar effects to other therapies approved, my valuable than the others because it is able to be delivered to patients [age complications, and the more they stand to benefit." – Provider organization
	Positive 15.9%	"Compared to [Product X], [Product Y] does not require regular [X route] adm favorable for patient quality of life. Additionally, roughly 4 years after receiving lower than that of [Product X]. This product has a real potential to decrease other comparators." – PBM organization
		"Cost of [Product X] is incredibly high up-front. Although in the long term you product being cost-effective, the up-front hit is a big one." – MCO
	Mixed 8.5%	"Based on the estimated price tag of [Product X], I cannot give it more econ highly effective across all [disease X] and has a plethora of real-world data. of [Product Y] to come close to the estimated cost of [Product X]. Patients m long term." – Other organization

n=283. Note: The sum of percentages may not equal 100% due to rounding. ^oOpen-ended responses have been blinded to product mentions or other product-specific characteristics. Other organization types include respondents from organizations such as academia, government, and consultants.

Q: What is your current overall perception of the economic value of each of the following products (based on the assumption of price parity with the primary comparator if a product was pre-approval)? Why did you rate economic value as you did? Key: MCO – managed care organization; PBM – pharmacy benefit manager.

out also the [administration] creates a situation niums." – MCO

atior

e same, I view the total economic value as

is that the drug could reach upwards of [X

y opinion is that [Product X] is just a notch more ed X]. The younger these patients, the fewer the

ministration. [Product Y] is a [X therapy] that is ing [Product Y], overall drug costs begin to be medical costs and complications compared f

ou could likely make a good argument over this

phomic value than [Product Y]. [Product Y] is a. Although highly priced, it would take years may not even have the same insurance [in the]

Figure 5: Distribution of overall sentiment by organization type^a



N=563. Note: The sum of percentages for each organization type may not equal 100% due to rounding. The percentages for other organization types reflect an average across multiple organizations; therefore, the sum of percentages may be greater than 100%. °Figure reflects aggregate sentiment data for clinical efficacy and economic value. Other organization types include respondents from organizations such as academia, government, and consultants.

Q: What is your current overall perception of the [clinical efficacy/economic value] of each of the following products (based on the assumption of price parity with the primary comparator if a product was pre-approval)? Why did you rate [clinical efficacy/economic value] as you did? Key: MCO – managed care organization; PBM – pharmacy benefit manager.

Figure 6: Distribution of sentiment for clinical efficacy by organization type



n=280. Note: The sum of percentages for each organization type may not equal 100% due to rounding. The percentages for other organization types reflect an average across multiple organizations; therefore, the sum of percentages may be greater than 100%. ^aIncludes respondents from organizations such as academia, government, and consultants.

Q: What is your current overall perception of the clinical efficacy of each of the following products (based) on the assumption of price parity with the primary comparator if a product was pre-approval)? Why did you rate clinical efficacy as you did? Key: MCO – managed care organization; PBM – pharmacy benefit manager.

Figure 7: Distribution of sentiment for economic value by organization type



n=283. Note: The sum of percentages for each organization type may not equal 100% due to rounding. The percentages for other organization types reflect an average across multiple organizations; therefore, the sum of percentages may be greater than 100%.

^aIncludes respondents from organizations such as academia, government, and consultants. Q: What is your current overall perception of the economic value of each of the following products (based on the assumption of price parity with the primary comparator if a product was pre-approval)? Why did you rate economic value as you did? Key: MCO – managed care organization; PBM – pharmacy benefit manager.

References: 1. Bohr A, Memarzadeh K. The rise of artificial intelligence in healthcare applications. In: Bohr A, Memarzadeh K, eds. Artificial Intelligence in Healthcare. Academic Press; 2020;25-60. **2.** Zunic A, Corcoran P, Spasic I. Sentiment analysis in health and well-being: systematic review. JMIR Med Inform. 2020;8(1):e16023.

Figure 8: Clinical efficacy ratings by sentiment type



n=280. Note: Standard error bars reflect how reliable the sample means are for each sentiment type; smaller standard errors suggest that the sample mean is a more accurate reflection of the true population mean. Standard error of the mean was calculated by dividing the standard deviation for each sentiment type by the root of the sample size for that sentiment type.

Q: What is your current overall perception of the clinical efficacy of each of the following products (based on the assumption of price parity with the primary comparator if a product was pre-approval)? Rate from 1-5: 1 = much less favorable; 2 = somewhat less favorable; 3 = equal to; 4 = somewhat more favorable; 5 = more favorable.

Figure 9: Economic value ratings by sentiment type

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2.45	2.84	3.13	2.95
Negative	Neutral	Positive	Mixed

n=283. Note: Standard error bars reflect how reliable the sample means are for each sentiment type; smaller standard errors suggest that the sample mean is a more accurate reflection of the true population mean. Standard error of the mean was calculated by dividing the standard deviation for each sentiment type by the root of the sample size for that sentiment type.

Q: What is your current overall perception of the economic value of each of the following products (based on the assumption of price parity with the primary comparator if a product was pre-approval)? Rate from 1-5: 1 = much less favorable; 2 = somewhat less favorable; 3 = equal to; 4 = somewhat more favorable; 5 = more favorable.

Study limitations

- Results were reported in aggregate and may not fully represent HCDM sentiments regarding any particular product. As this research only evaluated survey responses for 3 CGT products, caution should be used in generalizing the results to all CGTs.
- Inherent limitations of the sentiment analysis technology, including inaccuracies due to insufficiently labeled data or complex sentences, should be considered when reviewing the results.
- This research reflects the perspectives of HCDMs identified from users of FormularyDecisions; other user types (eg, patients, manufacturers) were not represented in this survey.
- The respondent sample had greater representation from MCOs, PBMs, and provider organizations, which could affect generalizability of the results across all types of organizations and HCDMs.
- Because all respondents voluntarily completed the survey, voluntary response bias may exist, and survey results may overrepresent respondents with stronger interest in payer-manufacturer partnerships.

Conclusions

- The results suggest that NLP sentiment analyses have utility in rapidly evaluating qualitative HCDM data.
- Sentiment for the 3 CGTs supports that there are varied perceptions across HCDMs regarding clinical efficacy and economic value and that these insights may vary by organization type.
- While overall sentiment varied across the evaluated CGTs, sentiment was slightly more negative and neutral compared to positive. When evaluating clinical efficacy, providers and MCOs appear to have a relatively more positive sentiment, while MCO, PBM, and provider perceptions of economic value appear to be largely negative.
- As HCDMs continue to provide feedback for CGTs, manufacturers should consider innovative methods for timely and targeted assessments to identify and address barriers that may impact successful commercialization and patient access.



Presented at: AMCP 2023 Annual Meeting, March 21–24, 2023; San Antonio, Texas. Direct questions to Breyanne Bannister at Breyanne.Bannister@xcenda.com This research was funded by Xcenda.

0.0% 20.0%

Other^a