

# Assessing Shifts in Payer Perspectives on Expedited Approvals, 2018 vs 2021: A Cross-Sectional Study Aligning Drug Development and Regulatory Approvals



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## Background

- There are four pathways through which the FDA can allow for earlier approval of drugs: (1) fast track designation, (2) breakthrough designation, (3) accelerated approval, and (4) priority review.
- FDA utilized at least one expedited program to speed approval for 74% of all novel drugs approved in 2021.<sup>i</sup>
- The evidence desired by payers for formulary decision-making may differ from FDA approval criteria when approving drugs via expedited pathways.<sup>ii, iii</sup>
- This study captures stakeholder concerns across formulary decision-making; health care resource utilization; real world evidence; drug development; and patient access.

## Objectives

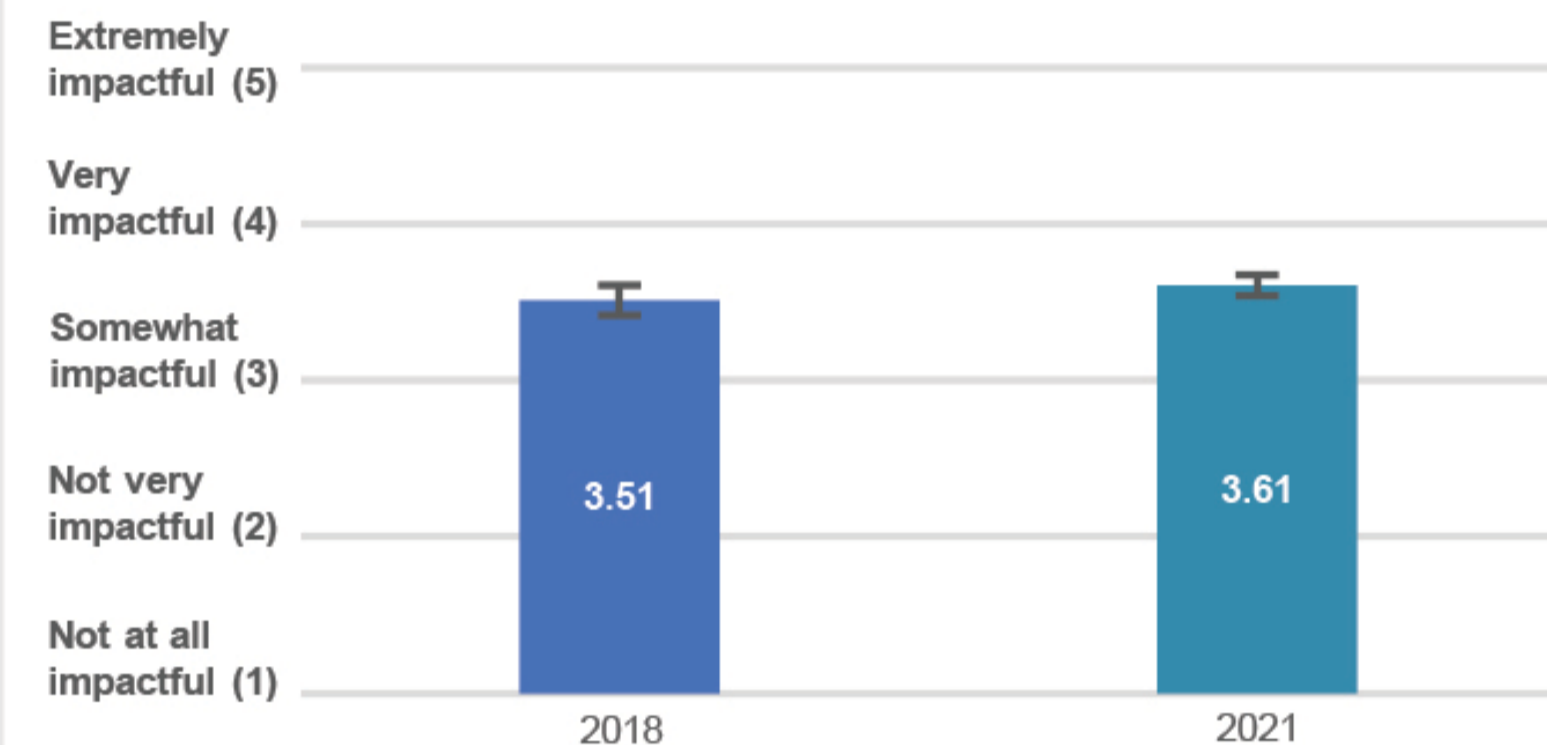
- To assess how payers' perspectives about accelerated drug approvals have shifted in the past three years.
- To identify evidence gaps to provide guidance on improving the expedited approval process.
- To ascertain treatment outcomes valued by payers, so that links from surrogate endpoints to meaningful outcomes are better understood by all stakeholders in drug development, regulatory approval, and formulary decision-making.

## Limitations

- The respondents' level of expertise and awareness cannot be verified or proven. Caution is advised when generalizing the survey results.
- Measures were taken to ensure an even distribution in the demographics. However, the results may be skewed towards the views of specific groups due to different response rates and participation bias.
- The survey design included many closed-ended questions with prepopulated responses.

## Results

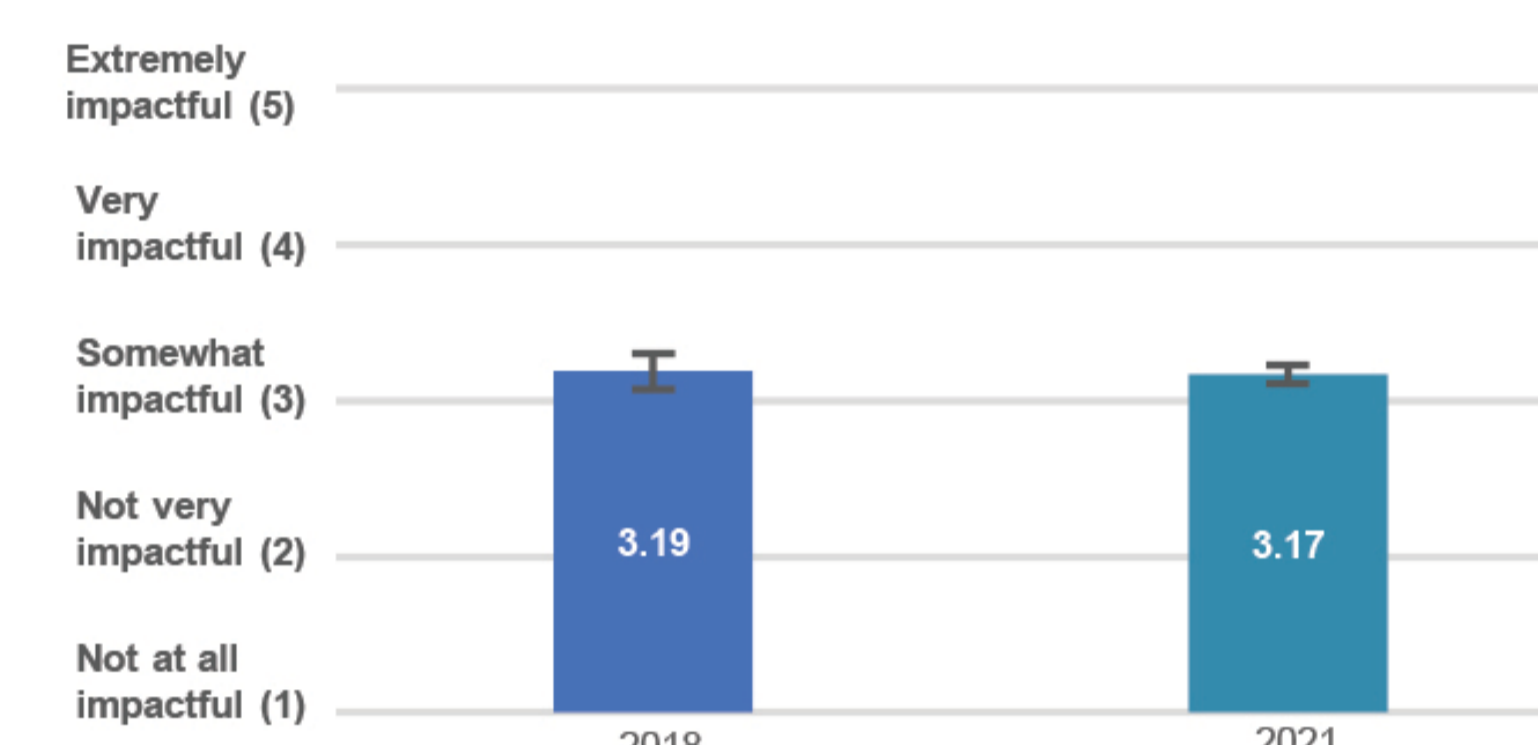
**Figure 1**



Statistical test:  $t(218) = -.78, p = .44, d = -.11$   
Notes: Respondents that answered "Not sure/I don't know" were removed from the analysis. All respondents that endorsed a work organization type of "other" were also removed. Averages were presented across years.

**Figure 1:** How *impactful* do you think accelerated drug approvals is to the future of healthcare? (1=not at all impactful, 5=extremely impactful)

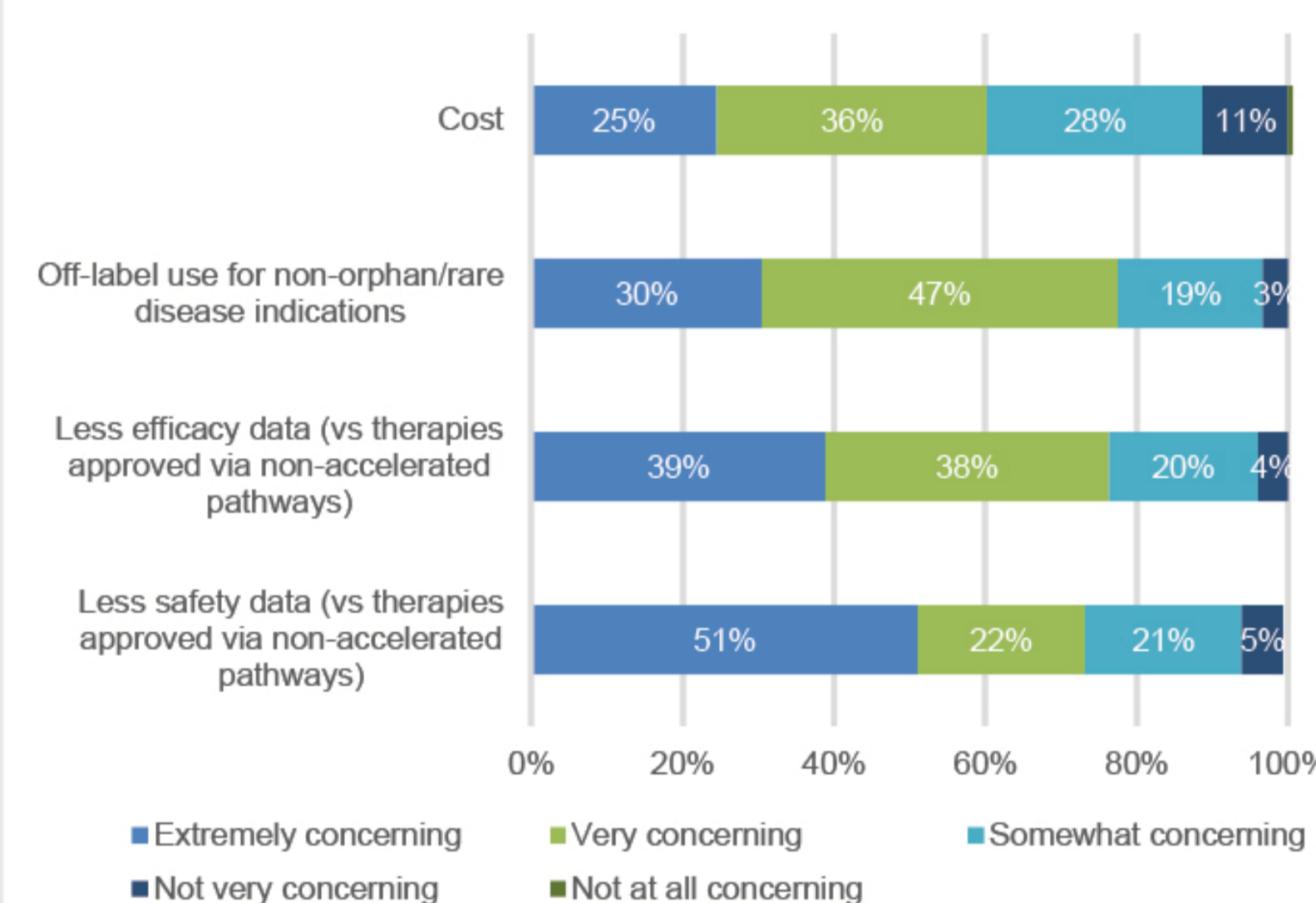
**Figure 2**



Statistical test:  $t(218) = .17, p = .87, d = .02$   
Notes: Respondents that answered "Not sure/I don't know" were removed from the analysis. All respondents that endorsed a work organization type of "other" were also removed. Averages were presented across years.

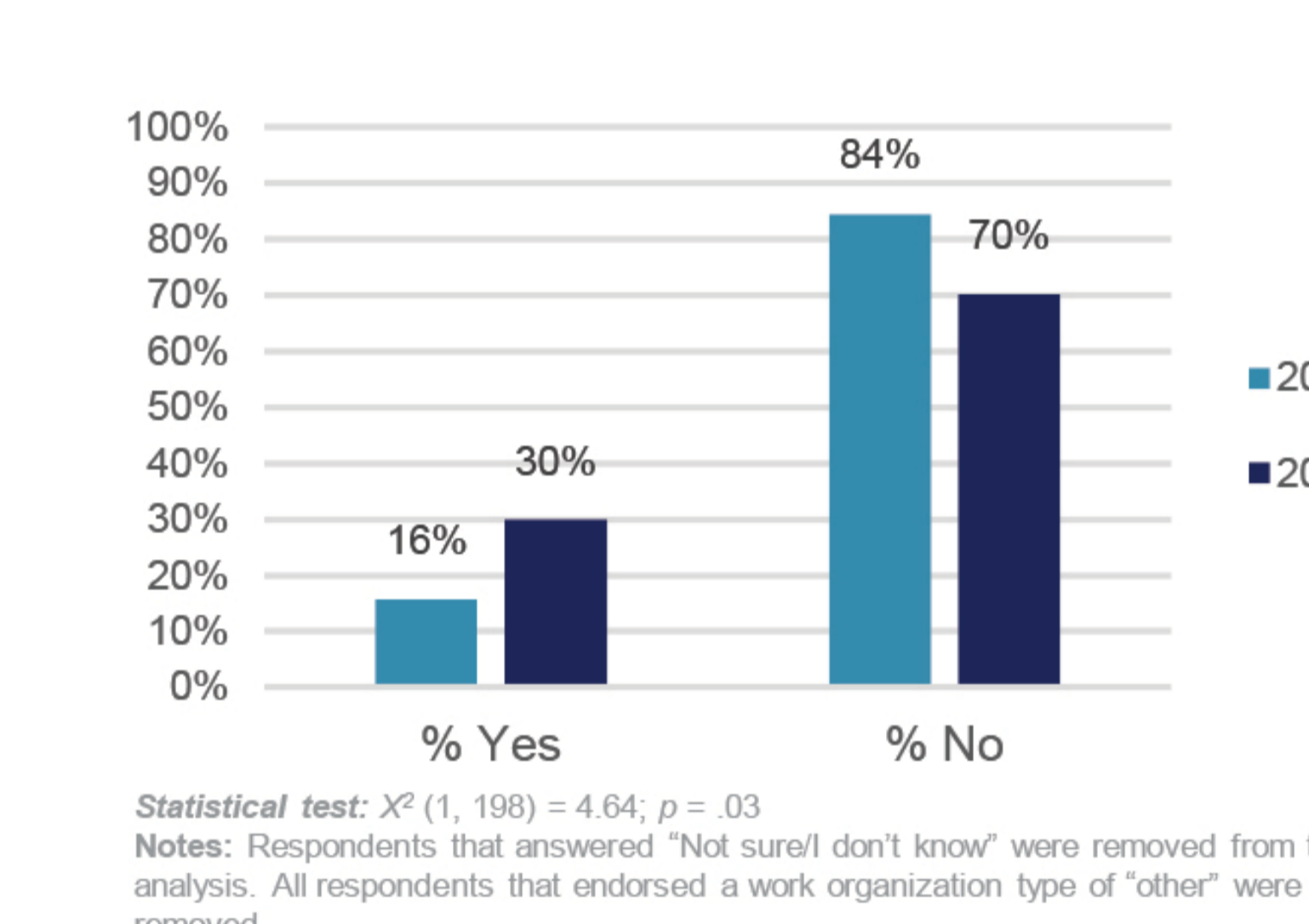
**Figure 2:** How would you rate the *need* for accelerated drug approval? (1=not at all necessary, 5=extremely necessary)

**Figure 3**



**Figure 3:** How *concerning* are each of the following with respect to accelerated drug approval? (1=not at all concerning, 5=extremely concerning)

**Figure 4**



**Figure 4:** Does your organization have a separate and/or *expedited review process* for therapies receiving accelerated drug approval?

Statistical test:  $\chi^2(1, 198) = 4.64, p = .03$   
Notes: Respondents that answered "Not sure/I don't know" were removed from the analysis. All respondents that endorsed a work organization type of "other" were also removed.

## Discussion and Conclusions

- The two surveys show moderate and consistent agreement among payers that accelerated drug approvals are very or extremely impactful to the future of health care. In 2021, 68% rated "very" or "extremely" impactful, vs. 8% who rated "not at all" or "not very" impactful. This roughly reflects the number of respondents who felt the same in 2018.
- Although payers recognize the *impact* of expedited reviews, they do not feel as strongly about the *need* for these pathways. Only 1/3 of all survey respondents rated the need for accelerated drug approval as "very" or "extremely" necessary in 2021. 14% felt that expedited reviews were "not at all" or "not very" necessary. This is consistent from 2018.
- Payers are deeply interested in more evidence, particularly around *safety* and *efficacy*. Three out of four managed care decision-makers were concerned with insufficient safety and efficacy data. This level of concern was consistent across years ( $p$ 's > .05).
- Payers were significantly more likely to have a separate review for therapies receiving accelerated approval in 2021 ( $p < 0.05$ ).
- Of note, a lack of *clinically meaningful outcomes* was also one of the most concerning evidence gaps among the 2021 cohort. Mapping links between surrogate endpoints and meaningful outcomes to inform formulary decisions, and to discern potential clinical impact of relevant surrogate markers, is extremely challenging.
- These insights on payer needs and current barriers for therapies approved through expedited reviews can assist in identifying potential solutions to address evidence gaps and reduce the financial uncertainty for these products.

## Methods

- This cross-sectional study consisted of a web-based survey with 22 multiple choice & open-ended questions.
- Four questions were repeated from the AMCPF Trends in Healthcare Survey (2018) to assess whether and how payers' perspectives have evolved on the topic of FDA expedited approvals.<sup>v</sup>
- Several questions explored payer reactions to recommendations recently issued by ICER<sup>v</sup> & Evidera.<sup>vi</sup>
- The 2021 survey instrument was circulated to pilot respondents ( $n=8$ ) to assess content and face validity.
- The 2021 survey was fielded from Sept. 22-Oct. 4. Fig. 1 presents employers of respondents to both surveys.
- Recruitment tactics, including a limited honorarium for validated respondents, were repeated from 2018 survey.
- Inferential and descriptive statistics were used to analyze the data. The independent t-test was utilized to compare 2018 and 2021 cohorts.

Work Organizations	2018 Survey n (%) n = 71	2021 Survey n (%) n = 159	$\chi^2, p$ -value
Managed care organization or health plan	43 (61%)	75 (47%)	$\chi^2 = 3.83, p = .43$
Pharmacy benefit management organization	19 (27%)	51 (32%)	
Specialty pharmacy	8 (11%)	10 (6%)	
Integrated delivery network	10 (14%)	11 (7%)	
Other	5 (7%)	12 (8%)	

**Figure 5**

## References

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