

Patient Outcomes and Preferences Regarding Hemophilia Treatment within a Commercially Insured Population: A Prospective Survey

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Introduction

- Hemophilia is a rare disease however is among the top 10 diseases of pharmacy benefit spending and the top 5 for medical benefit spending¹.
- Blue Cross Blue Shield of North Carolina (Blue Cross NC) has made advancements in balancing access and medication costs for hemophilia treatments, including utilization management programs and establishing the Blue Cross NC Hemophilia Specialty Pharmacy Network.
- The World Federation of Hemophilia guidelines recommend incorporating patient assessments that encompass aspects such as body structure and function, daily activities, and social interactions².
- The active involvement and dedication of patients in making treatment decisions play a crucial role in the direction of medical advancements and improvement of real-world outcomes.

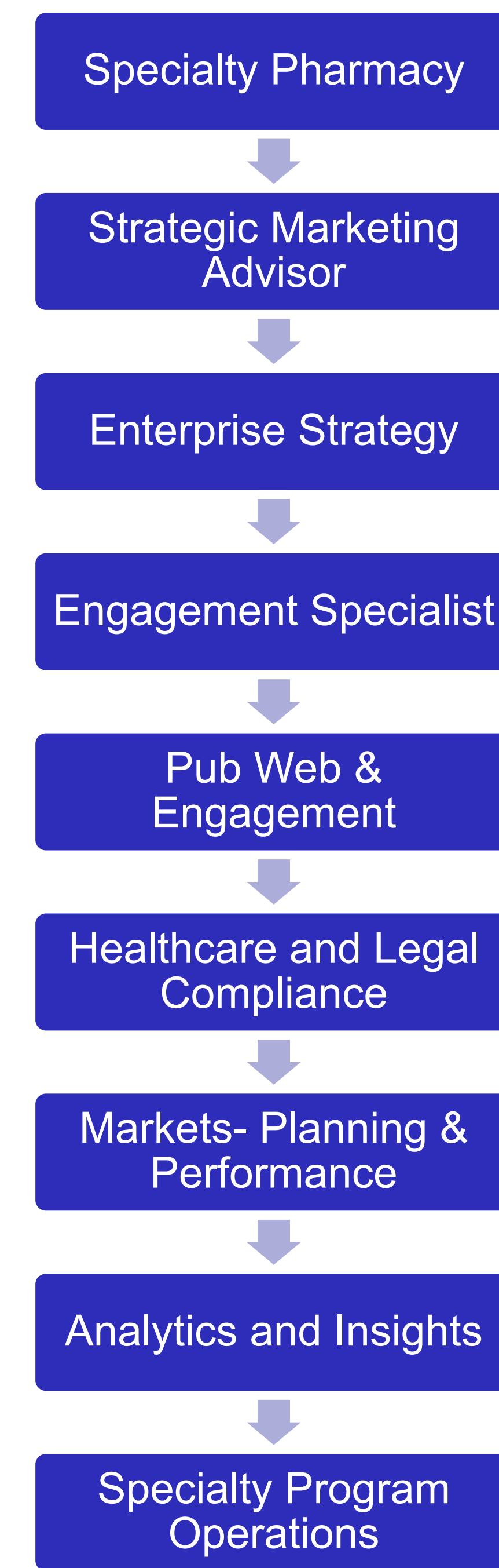
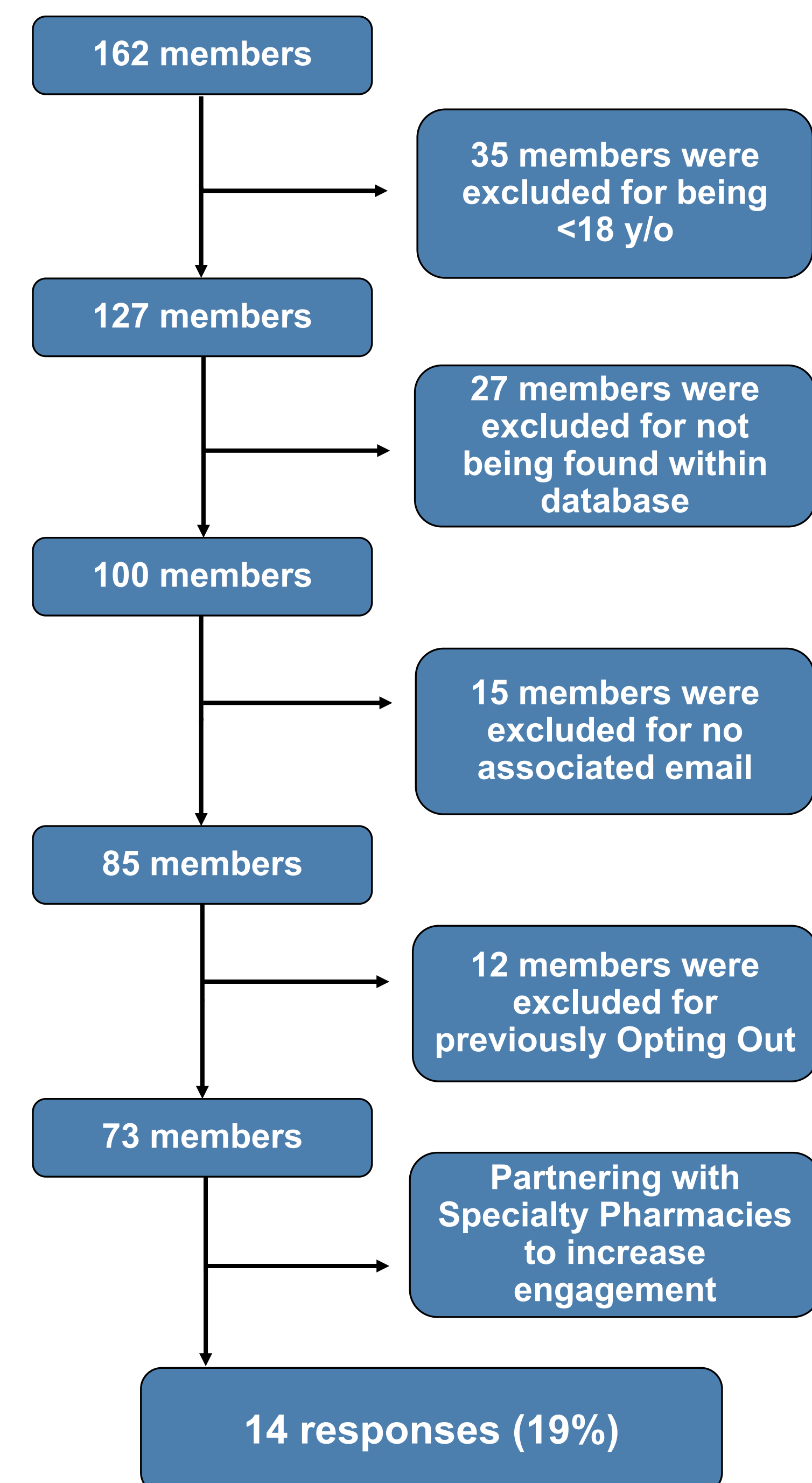
Objective

- Design, distribute and analyze a patient-facing survey to understand Blue Cross commercially insured patient outcomes regarding their current treatment and preferences regarding future treatments.

Methods

Design: A prospective survey study of hemophilia patients within the Blue Cross NC commercially insured population was conducted in August 2023. The survey population was identified by a retrospective review of medical and pharmacy claims data between April 1st, 2022, and April 30th, 2023, (n=162). After collaboration with Survey Engagement team and developing the campaign survey, population was narrowed to seventy-three patients. After pre-determined inclusion and exclusion criteria was met, the final study population received the prospective survey (Figure 1).

Figure 1: Campaign report for population **Figure 2: Teams involved in design of survey**



Survey Sections:

The ten-question survey had four sections:

- 1) Demographics
- 2) Outcomes with current therapy
- 3) Future therapy preferences
- 4) Optional open-ended text space for patients to share any concerns regarding their treatment or preferences

Analysis:

Survey responses were assessed using descriptive statistics.

The average ranking of the selections are stated in Table 2, The sum was divided by number of choices.

Results

Figure 3: Disease Severity Among Patients

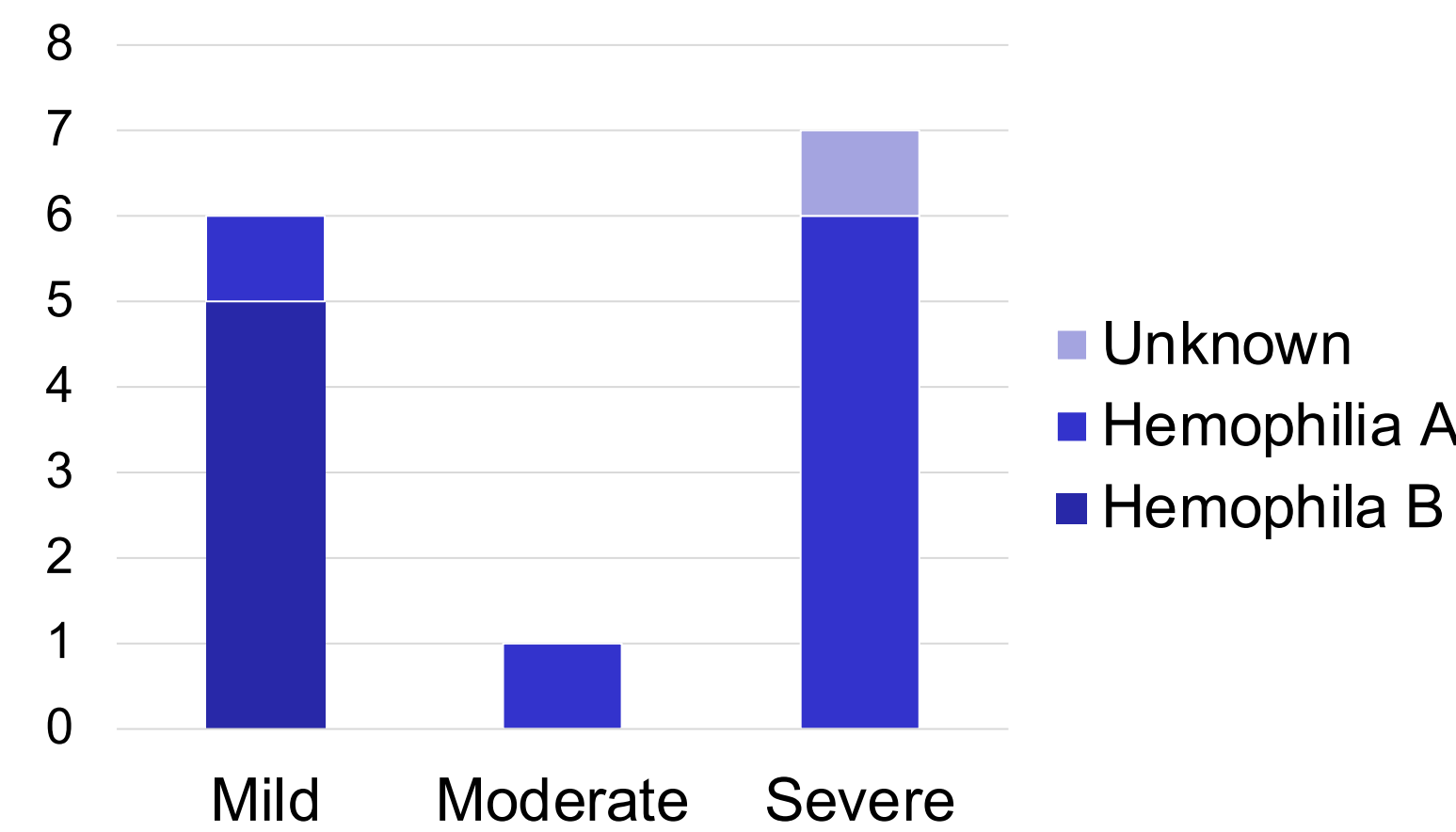


Figure 4: Likelihood of Patients Discussing Treatment Options with Doctors

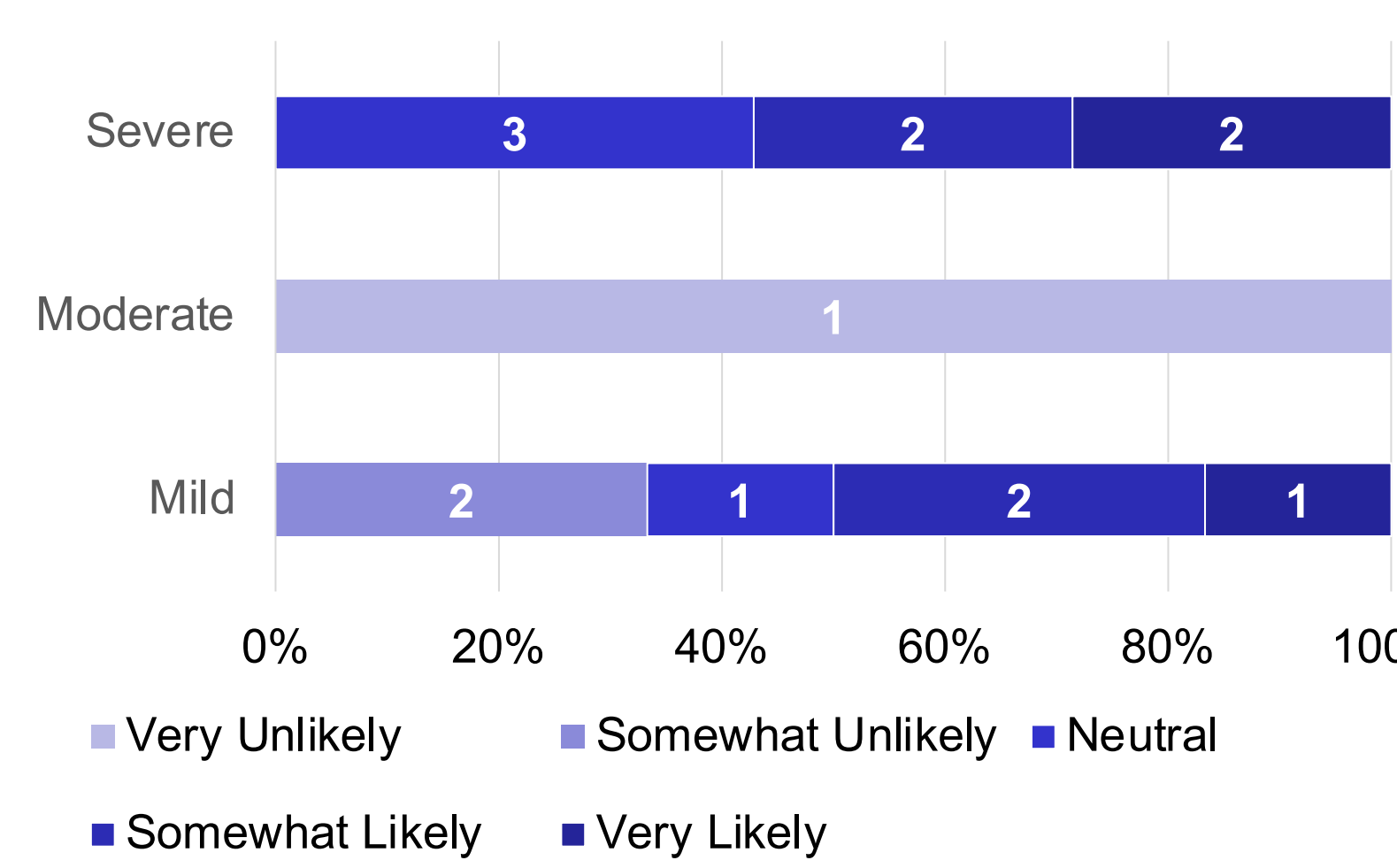


Figure 5: Patients Reported Quality of Life Outcomes Past 3 Months

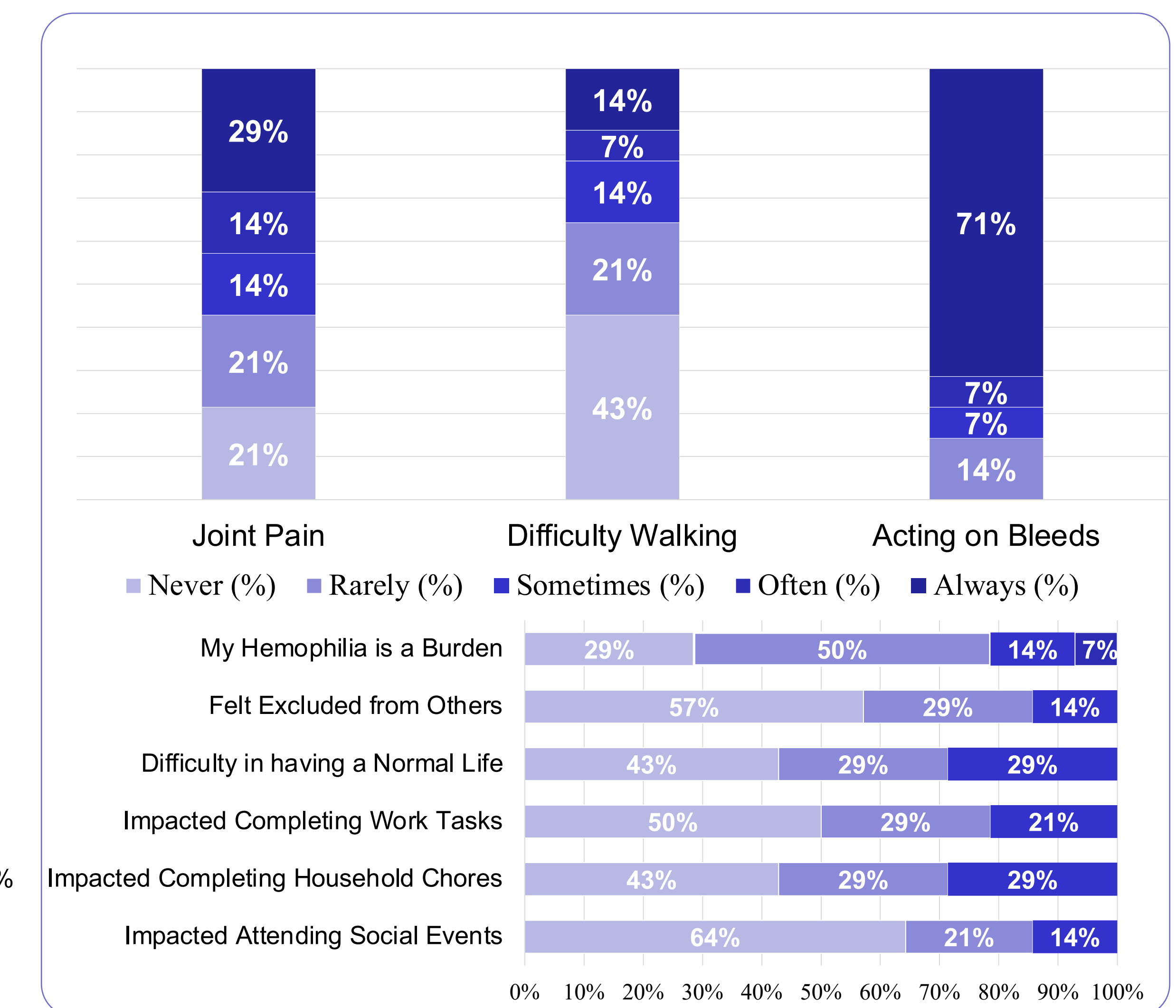


Table 1: Patient's Recorded Bleeds

# of Bleeds	Responses	Hospitalizations
1	2	0
2	0	0
3	2	2
4 or more	1	0
Total	6	2

Table 2: Patient's Preferences

Patient Preferences	Mild Patients (Rank Avg)	Severe Patients (Rank Avg)
Administration Frequency	1.8	2.4
Bleeding Frequency	2.8	2.0
Adverse Events Frequency	3.8	4.0
Impact on Daily Life	2.2	2.3
Impact on Mental Health	4.3	4.3

Key Takeaways:

- The likelihood of a patient discussing treatment options with their doctors was 3.3 on average, which lies between Neutral (3) and Somewhat Likely (4). Severe patients reported higher average of 3.85 in comparison to mild/moderate patients at a 3 average.
- Out of nine categories in Figure 5, only 1 (Joint Pain) did not have the majority (>51%) of patients within the Never/Rarely selection stating that these patient's quality of life is managed within their current treatment.
- The Impact on Daily Life (Avg 2.2, St Dev 1.0) and Administration Frequency (Avg 2.2, St Dev 0.94) were the highest ranked preferences when considering treatments across all three severities.

Conclusions/Future Directions

Results demonstrated a common trend between the severities of hemophilia and the reported quality of life. Majority of patients (>51%) rarely or never experienced the outcomes surveyed in the past three months. All severe patients within the survey stated that they would be inclined to discuss their condition with their doctor, compared to those affected mildly and moderately, of which at least one patient would not be inclined (Figure 4). Analysis of the mild and severe categories showed differences in preferences, as severe patients stressed Bleeding Frequency, while mild patients stressed Administration Frequency. Our survey was novel but had a low response rate within the first three weeks. Collaboration with our in-network Specialty Pharmacies increased our response rate to 19% (14/73). Additional research is necessary to obtain results of greater importance. One way to expand our reach would be to collaborate with other payers supported by Prime Therapeutics. Pharmacy benefit managers and payers should consider real-world evidence for use in future formulary management as new hemophilia treatments become approved.

Limitations

- No incentive was provided to patients for completing the survey; response rate may have been higher with inclusion of an incentive.
- Survey length may have caused low response rates, there were entries that were initiated but not submitted. The average completion time was 455s, around 7.5 minutes
- Patients living with hemophilia may become desensitized to the implications of hemophilia, which may skew the data of patient outcomes being less severe.

References

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Acknowledgements



Thank you to Pfizer, Inc. for funding the AMCP Foundation Managed Care Internship Program.

Thank you to the Specialty Pharmacies in assisting the distribution of the survey.