

Real-World Experience and Strategies to Enhance the Palatability of the Combination Sodium Phenylbutyrate and Taurursodiol for the Treatment of Amyotrophic Lateral Sclerosis



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BACKGROUND

- Sodium phenylbutyrate and taurursodiol (PB&TURSO) is an oral, fixed-dose, combination medication with full approval to treat adults with amyotrophic lateral sclerosis (ALS) in the United States and approval with conditions for the treatment of ALS in Canada^{1,2}
- In the CENTAUR trial, administration of PB&TURSO significantly slowed functional decline compared with placebo in people living with ALS (PLWALS)³⁻⁵
- PB&TURSO was generally well tolerated in the trial with an acceptable safety profile; gastrointestinal events occurred with greater frequency in the PB&TURSO group^{3,6}
- PB&TURSO can have a bitter taste to some PLWALS,⁶ which may compromise adherence

OBJECTIVE

- To explore and evaluate the real-world taste experiences of PLWALS in the United States being treated with PB&TURSO and the coping strategies used to potentially enhance PB&TURSO palatability

METHODS

- A 20-minute online survey was developed by Amylyx Pharmaceuticals in collaboration with PLWALS, an ALS clinician, and a food scientist
- Respondents included PLWALS enrolled in the Amylyx Care Team (ACT)TM Support Program who had opted in for market research and who currently or previously received PB&TURSO by mouth
- Survey respondents received an honorarium from Amylyx Pharmaceuticals for participation
- Descriptive statistics were performed, and data were analyzed to generate themes

RESULTS

Participants

- A total of 150 PLWALS participated in the survey (Table 1)

TABLE 1. PARTICIPANT DEMOGRAPHIC AND CLINICAL CHARACTERISTICS

Clinical Characteristic	No. (%)
PB&TURSO experience	
Currently taking	111 (74)
Previously taken	39 (26)
Age (median), y	63
Sex	
Male	82 (55)
Female	68 (45)
Time since official ALS diagnosis	
≤6 mo	5 (3)
>6 mo to <1 y	30 (20)
>1 y to <2 y	57 (38)
>2 y to <3 y	23 (15)
≥3 y	35 (23)
Extent of disease progression	
Able to live independently	58 (39)
Limited ability to function independently	76 (51)
Serious difficulties, requiring full assistance	16 (11)

RESULTS (cont'd)

Perception of PB&TURSO Taste

- The majority of survey participants reported that PB&TURSO had an unpleasant taste when first starting on therapy
 - Most described the taste of PB&TURSO as “bitter”
- Thirty percent (n=45/150) of survey participants noted their perception of PB&TURSO taste improved over time, most within 1 month of initiating treatment

Impact of PB&TURSO Taste Perception on Adherence and Use

- The majority of participants reported that the taste of PB&TURSO did not meaningfully affect their willingness to take it every day as prescribed, with 53% noting it had no effect (Figure 1)
 - For the vast majority (87%), taste had no impact on their reported adherence to PB&TURSO⁶
- Almost all (95%) of the survey participants currently on therapy planned to continue taking PB&TURSO for at least the subsequent 3 months (Figure 2)
 - Median time on PB&TURSO was similar between those who were planning to continue taking PB&TURSO (7 months; range, 1-16 months) and those who were not planning to continue (8 months; range, 7-19 months)
 - Of the 5 participants who reported not planning to continue PB&TURSO, only 1 cited taste as a reason for discontinuing

FIGURE 1. IMPACT OF TASTE OF PB&TURSO ON WILLINGNESS TO TAKE AS PRESCRIBED (N=150)

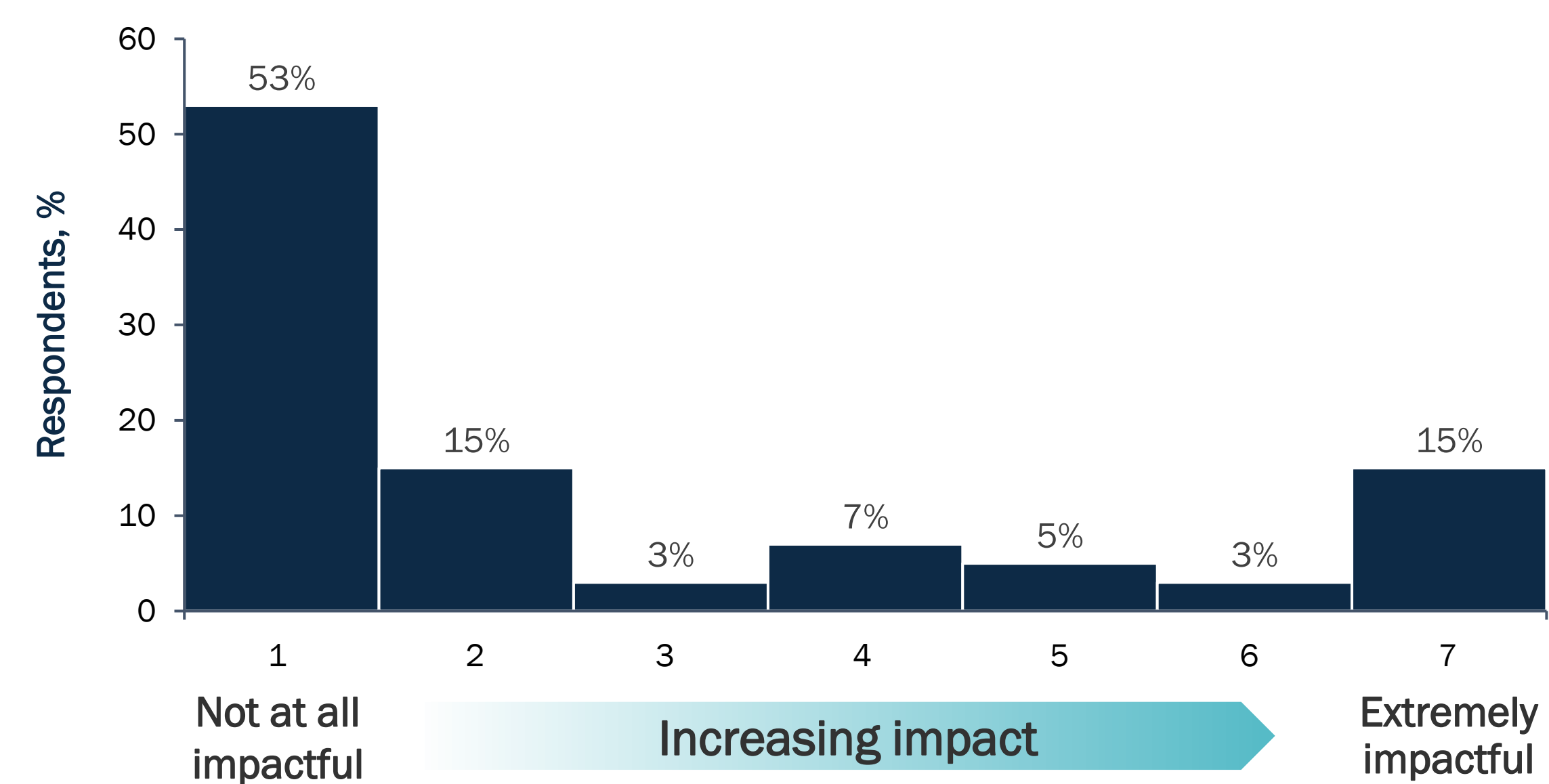
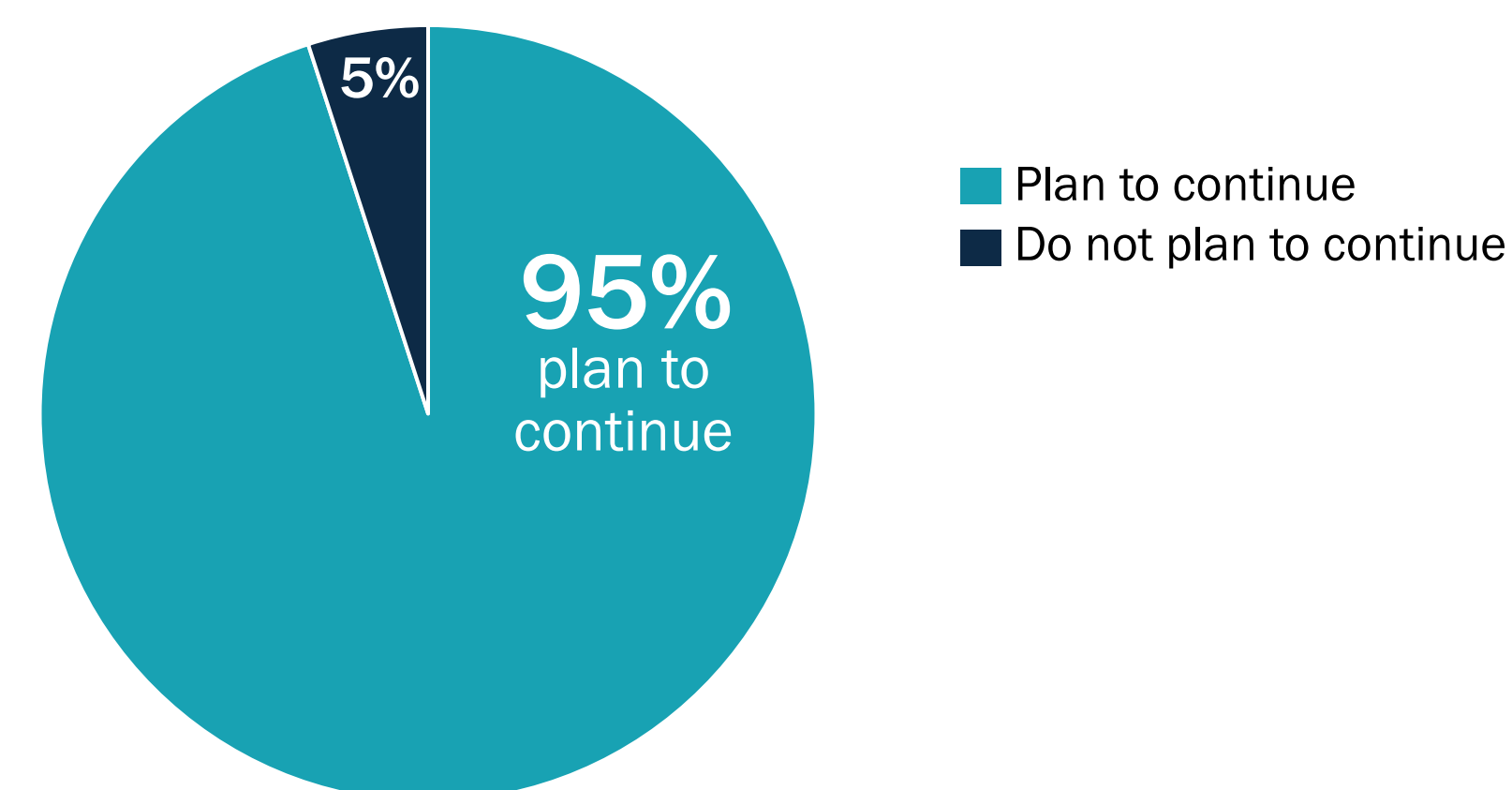


FIGURE 2. PLANS TO CONTINUE PB&TURSO FOR AT LEAST THE SUBSEQUENT 3 MONTHS^a



^aAmong participants who were currently receiving PB&TURSO at the time of the survey (n=111, including 96 currently taking PB&TURSO by mouth and 15 currently receiving PB&TURSO via feeding tube but who had previously taken it by mouth).

Acknowledgments

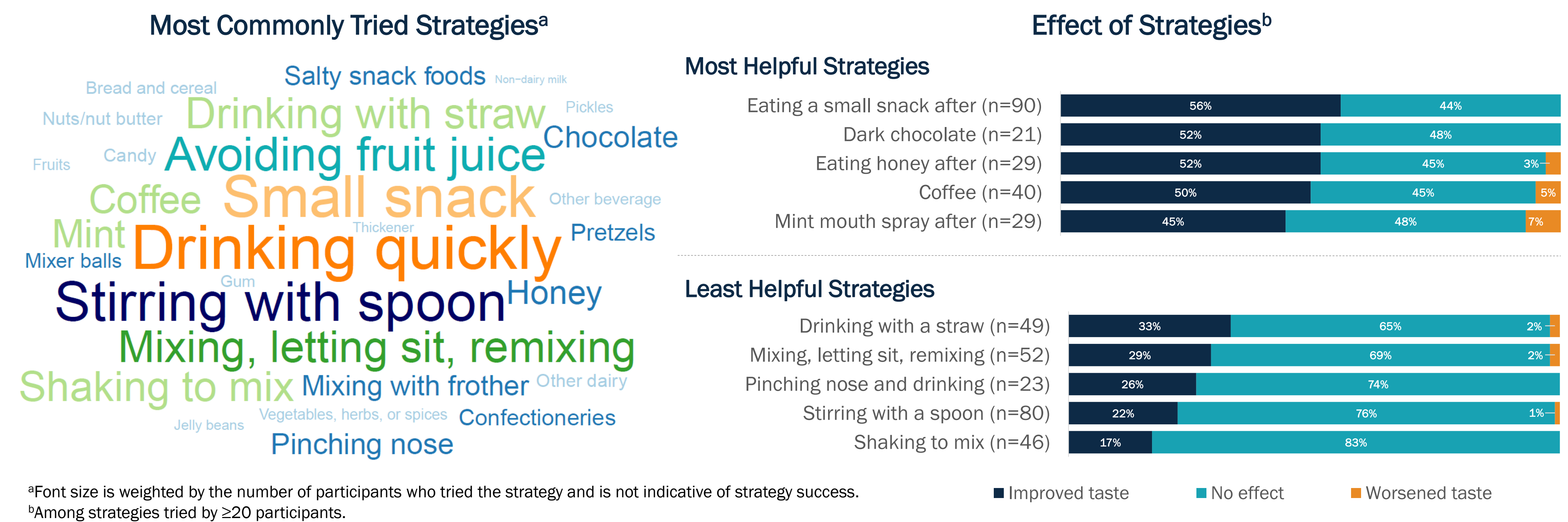
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RESULTS (cont'd)

Strategies to Enhance PB&TURSO Palatability

- Nearly all (96%) participants had tried ≥1 strategy to enhance the palatability of PB&TURSO
- The most commonly reported strategies are summarized in Figure 3, along with the most and least helpful of these strategies
- Individual responses to each strategy were variable
 - A single strategy could improve palatability for some individuals and worsen palatability for others
- Approximately two-thirds (66%) of participants reported finding a successful strategy that improved the palatability of PB&TURSO

FIGURE 3. STRATEGIES TRIED TO ENHANCE THE PALATABILITY OF PB&TURSO

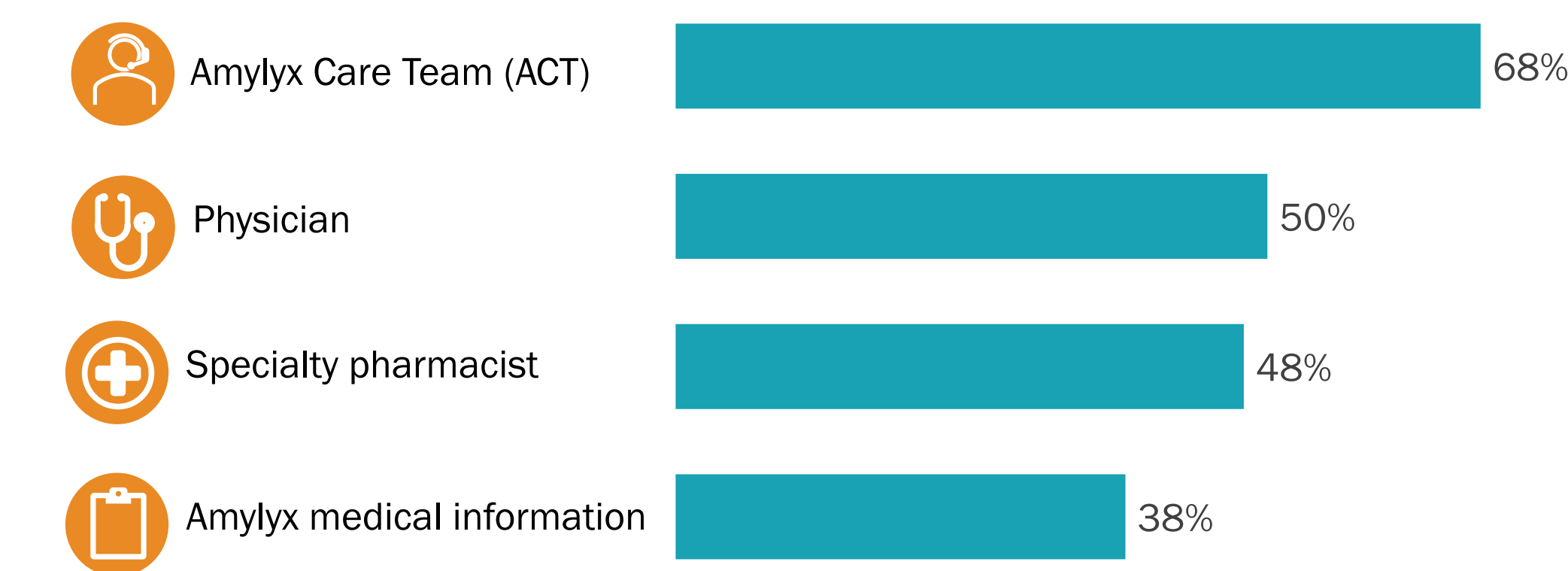


^aFont size is weighted by the number of participants who tried the strategy and is not indicative of strategy success.
^bAmong strategies tried by ≥20 participants.

Information Sources Relating to PB&TURSO Taste

- The most common resources that PLWALS reported using for information relating to PB&TURSO taste and strategies for enhancing PB&TURSO palatability included physicians (47%) and specialty pharmacists (42%), in addition to resources from Amylyx Pharmaceuticals (ACT, 62%; medical information, 38%)
- Nearly half of PLWALS who used physicians and specialty pharmacists as resources for information on PB&TURSO taste identified them as the most helpful sources for such information (Figure 4)

FIGURE 4. MOST HELPFUL RESOURCES FOR INFORMATION RELATING TO PB&TURSO TASTE^a



^aAmong the most commonly used resources (n≥50) overall, as determined only by those respondents who used each respective resource as a source of information on PB&TURSO taste.

Disclosures

- JT, KF, RY, and MM are full-time employees of and may have stock ownership in Amylyx Pharmaceuticals, Inc.
- LO and GP provide consulting services for Amylyx Pharmaceuticals, Inc.

CONCLUSIONS

- While the majority of survey participants reported perceiving the taste of PB&TURSO as unpleasant, perceptions of taste improved over time for nearly a third of individuals
- Taste did not appear to impact participants' willingness to take, adherence to, or planned future use of PB&TURSO, with nearly all reporting that they planned to continue PB&TURSO for at least the subsequent 3 months
- The majority of participants reported they were able to find a way to improve the palatability of PB&TURSO, though PLWALS may have to try a variety of strategies before finding what works for them
- The most commonly used and helpful sources of information regarding the taste of PB&TURSO and strategies to improve palatability included conversations with physicians, underscoring the importance of personal expectation setting and counseling
- New PB&TURSO formulations with improved palatability are being explored

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