

# Real-time and Short Recall of Flares in PNH, Captured through Longitudinal Home-Reported Outcomes

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## Objective

To compare retrospective flare reporting to real-time, patient-reported data captured via the Folia Health app, assessing recall sensitivity and specificity while characterizing flare duration, triggers, and daily life impacts.

## Background & Significance

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare hematological condition characterized by chronic symptoms including fatigue, hemoglobinuria, and risk of thrombosis that may significantly impact patient quality of life

- While the underlying hemolytic process is continuous, patients may experience symptom exacerbations
- Historically, these exacerbations have been captured via point-in-time patient-reported outcome measures, which may not fully characterize flare incidence and severity due to recall bias
- Understanding the concordance between real-time and retrospective flare reporting can inform optimal data collection strategies in rare disease research

## Methods

- **Participants:** 39 Folia Health users with PNH recorded data over a 5-month period
- **Data Collection:** Participants tracked their health via two distinct data sources in the Folia Health app:
  - Real-time home-reported outcomes (HROs), including flare tracking to capture flare onset, duration, triggers, and effects on daily life
  - Monthly retrospective surveys to recall flare instances over the prior month
- **Analysis:** Retrospective responses about flares were compared to data obtained via ongoing real-time tracking; sensitivity and specificity were calculated to assess concordance between the two measurement approaches

## Results

- Retrospective monthly surveys showed high sensitivity (94%) but low specificity (46%) relative to real-time flare tracking, indicating that *when* flare data is self-reported (immediately vs days/weeks after the event) affects *what* is captured
- Real-time tracking yielded rich data on flare characteristics, capturing a wide range of flare symptoms, triggers, and impacts to daily life
- Findings from real-time flare tracking data indicated that flare characteristics are heterogeneous in this PNH population

Figure 1. Flare Symptoms by Occurrence

Common vs Frequent, Among Top 10 Flare Symptoms

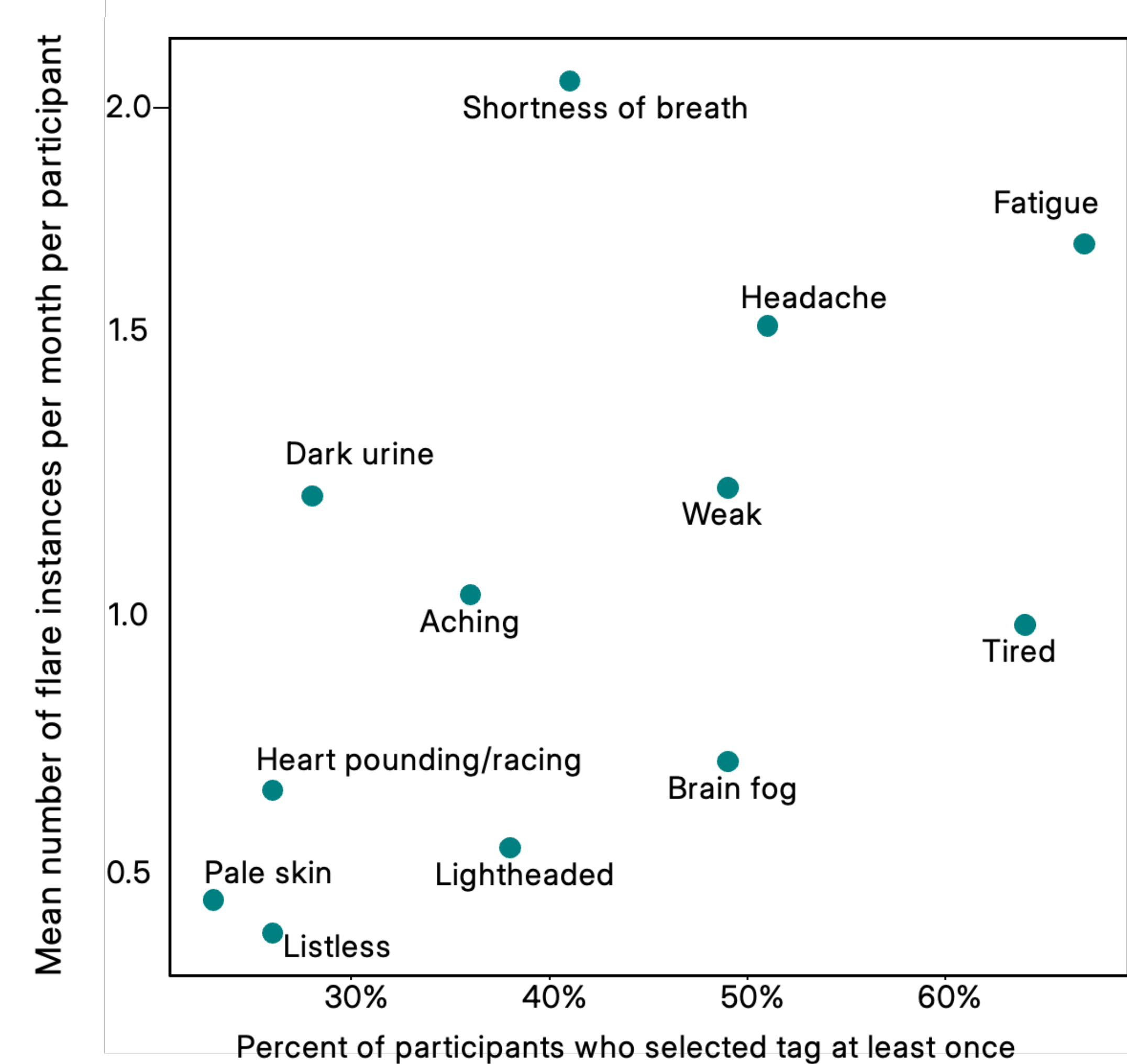


Figure 2. Flare Triggers by Occurrence

Common vs Frequent, Among Top 10 Flare Triggers

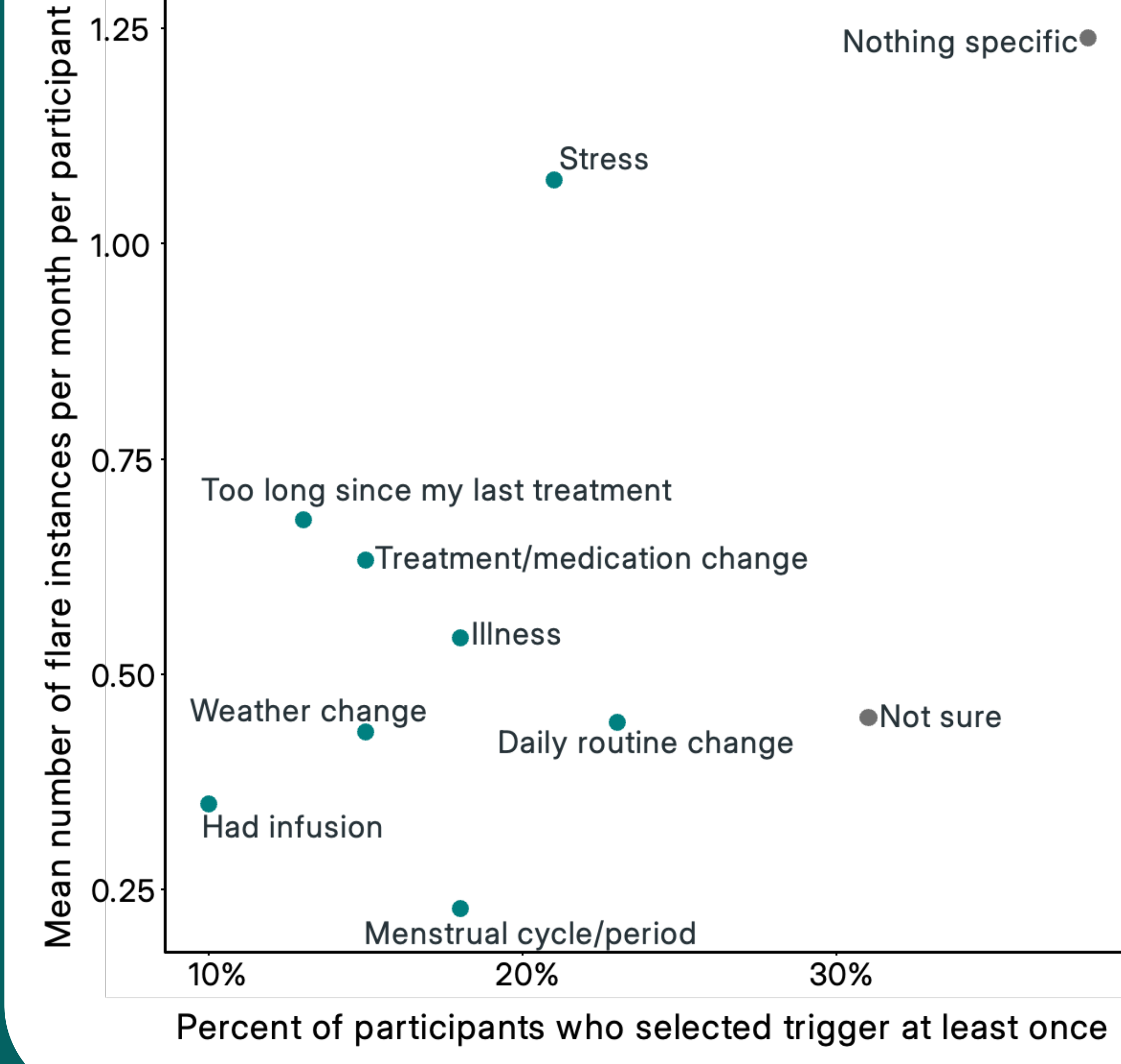


Figure 3. Flare Impact by Occurrence

Common vs Frequent, Among Top 10 Flare Impacts

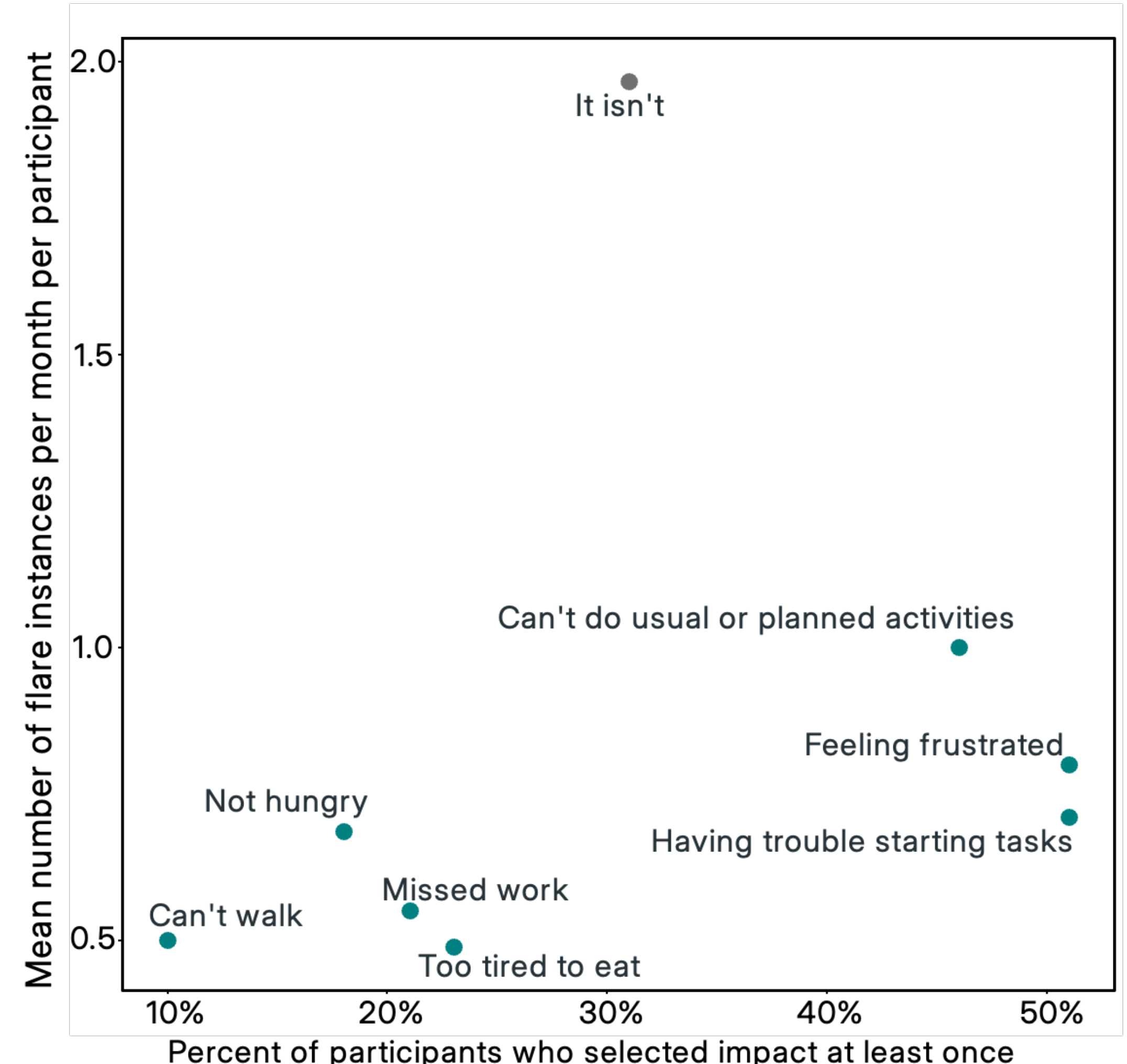


Figure 4. Confusion Matrix

Characteristic	N (%) or Mean (SD)
<b>Total N</b>	39
Non-responders to demographic survey	4 (10%)
Mean (SD) age	42.9 (14.5)
<b>Gender</b>	
Female	26 (67%)
Male	9 (23%)
<b>Race</b>	
White or Caucasian	28 (72%)
Black or African American	2 (5%)
More than one race reported	2 (5%)
Some other race	2 (5%)
Asian	1 (3%)
<b>Ethnicity</b>	
Not Hispanic or Latino	30 (77%)
Hispanic or Latino	4 (10%)
Prefer not to answer	1 (3%)

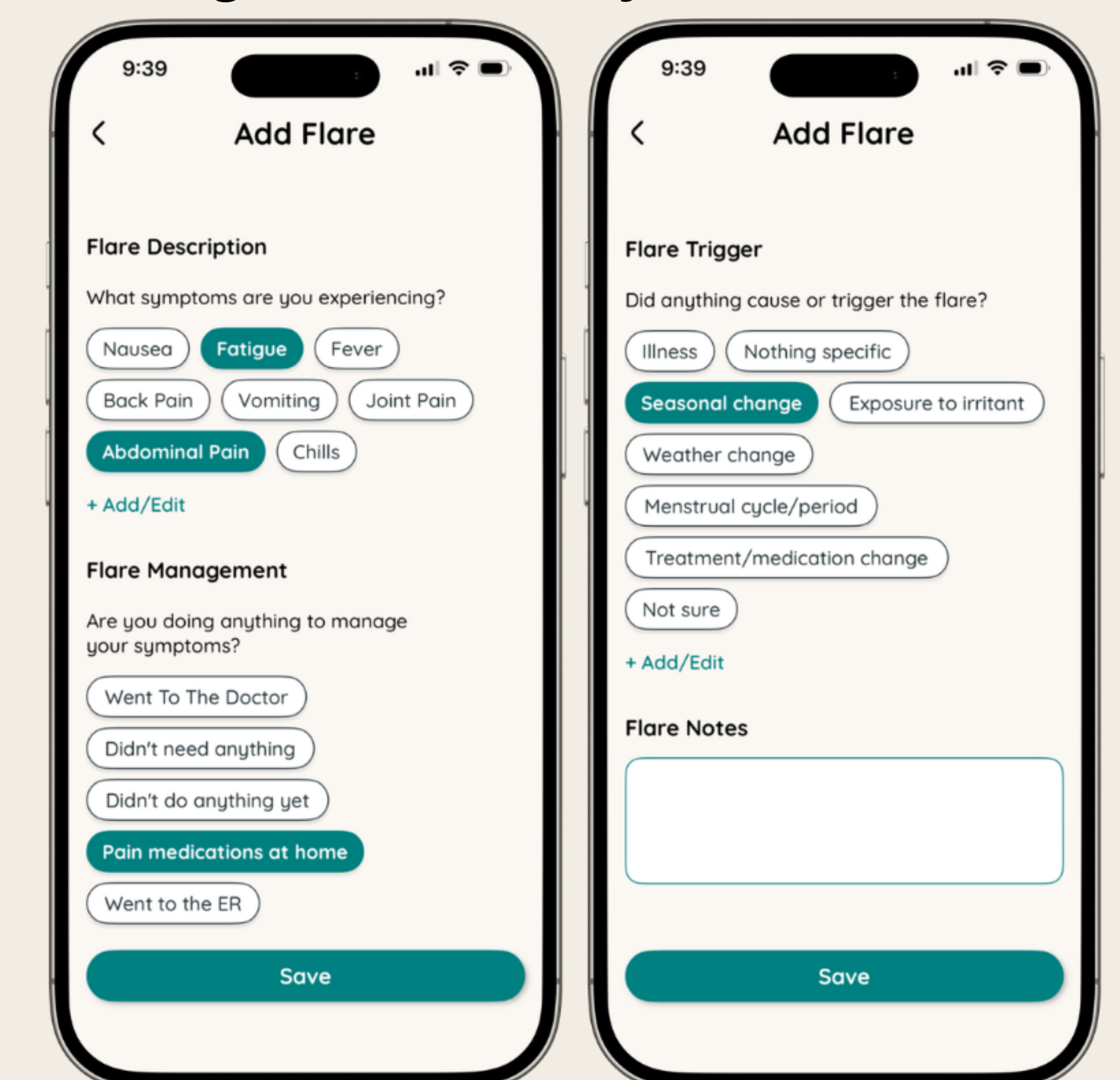
Confusion Matrix	
Flare (HRO = 1)	True Positive 58
Flare (HRO = 1)	False Negative 4
No Flare (HRO = 0)	False Positive 48
No Flare (HRO = 0)	True Negative 41

Predicted: Monthly Retrospective Survey Response  
 Sensitivity: 0.935 | Specificity: 0.461

## Key Findings & Conclusions

- Monthly retrospective and real-time reporting yield divergent data on flare incidence, with recall-based measures showing high sensitivity (94%) but lower specificity (46%). Future research should examine the reasons for differences between these two measurement approaches
- Real-time collection of PNH flare data captures detailed data on flare characteristics not available through monthly retrospective surveys
- Flare data captured in real time using a digital app approach may enable future analyses of treatment impact on core aspects of disease burden in PNH as well as in other rare diseases

Figure 5. Example Tagging & Flare Tracking Functionality



**Acknowledgments**  
 The authors want to thank all participants of this study. We are also grateful to Ally Hotz and Ariel Simon for their support in poster design.

