INTRODUCTION

- Systemic mastocytosis (SM), driven by the KIT D816V mutation, leads to debilitating and unpredictable symptoms and impaired patient quality of life (QoL).
- Assessing patient symptomology and impact of SM symptoms on QoL with valid SM-specific patient-reported outcome measures (PROMs) is critical for the evaluation of SM disease burden and treatment benefit.
- The development and validation of generic and disease-specific PROMs is a multi-year process requiring several clearly established steps.1-4

OBJECTIVES

- This review summarizes available SM PROMs, and profile the development, content, and application of these tools to aid in the selection of appropriate SM PROMs for use in clinical research and practice.

METHODS

- We conducted a structured review of peer-reviewed literature to identify SM-specific PROMs.
- PubMed, Ovid EMBASE, and clinicaltrials.gov were searched up to July 2023 to identify articles on the development and use of SM PROMs.
- For each PROM, we summarize the content, development, patient-reported outcomes, and use in clinical trials.

RESULTS

- We identified six SM PROMs: four symptom assessment tools and two QoL questionnaires.
- Two of the symptom assessment tools were SM subtype-specific (i.e., ISM, AdvSM); the other two were two QoL questionnaires, designed for all SM patients (Figure 1).1-7,8,9
- Each SM PROM underwent rigorous development and validation (i.e., reliability, validity, responsiveness). Robust interpretation guidelines and respondent burden varies by tool (Table 1).6,7,9
- The ISM-SAF is the only SM PROM that has been included in a registrational trial as a primary endpoint and is included in an approved product label.14 It is also a primary endpoint in an ongoing trial.7
- The AdvSM-SAF was included as an exploratory endpoint in two AdvSM clinical trials15,14; the MAS is an exploratory endpoint in an ongoing AdvSM trial.7
- SM PROM content, scoring, administration, adaptability, and use in clinical trials varied by measure (Table 2).
- The MC-QoL had fewer questions (n=27) and was used more commonly in randomized controlled trials (RCTs) than the MQLQ (n=49) (Table 2).1-12,13,14

CONCLUSIONS

- Many SM-Specific PROMs have been developed, validated, and used in clinical trials in the last several years; four quantity the severity of SM-specific symptoms and two assess patient QoL.
- The ISM-SAF has undergone rigorous validation and extensive interpretability analyses over many years, with regulatory input, to support an approved product label.
- The intended patient population, content, respondent burden, and interpretability of SM PROM data varies by tool and should be considered by clinicians and researchers to use in clinical practice, research, or quality initiatives.

Several SM PROMs have been developed to assess symptomology and quality of life (QoL).

The attributes, utility, and interpretability of existing SM PROMs vary widely and should be carefully considered prior to implementation in clinical trials.

References: