

A bespoke outcome measure is needed for symptom burden in people with idiopathic multicentric Castleman disease (iMCD).

Developing a Novel Outcome Measure to Assess Symptom Burden in Idiopathic Multicentric Castleman Disease: Protocol of an International Collaborative Project

Introduction: Idiopathic multicentric Castleman disease (iMCD) is a rare lymphoproliferative condition with a high symptom burden and health-related quality of life impacts. No validated condition-specific patient-reported outcome measure (PROM) exists to reliably assess symptom burden in iMCD and support research and clinical decision-making. The **i**diopathic **M**ulticentric **C**astleman Disease **S**ymptom **B**urden **S**tudy (**ISBUS**) is an international collaboration to develop a novel iMCD PROM involving the US, UK, Canada, Australia, and Brazil.

Methods:

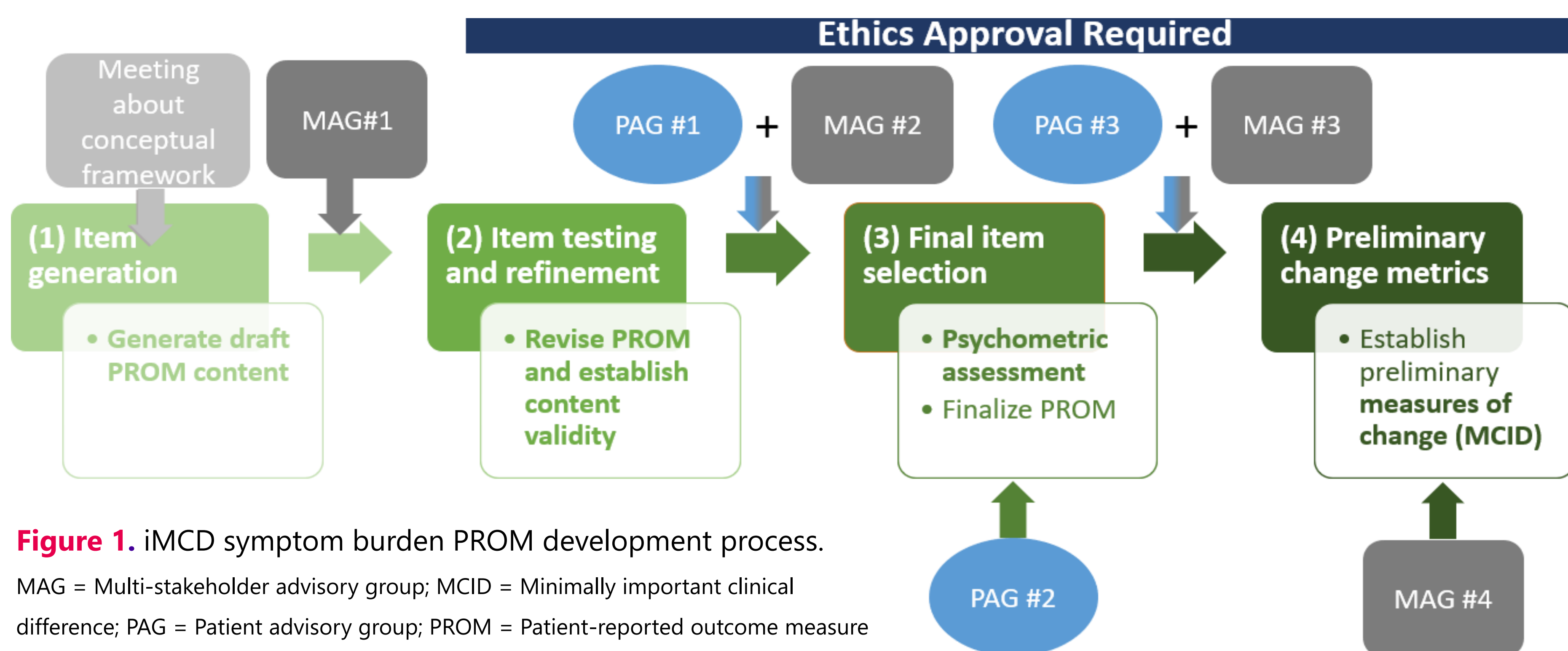


Figure 1. iMCD symptom burden PROM development process.

MAG = Multi-stakeholder advisory group; MCID = Minimally important clinical difference; PAG = Patient advisory group; PROM = Patient-reported outcome measure

ISBUS is an observational, non-interventionist study (NCT05995834). It involves mixed methodologies and has four stages (**Figure 1**). Key advisory groups include a dedicated patient advisory group (PAG) and multi-stakeholder advisory group (MAG) including clinical experts. Stage 2 (cognitive interviewing) is concluding as of October 2024.

Results: Forty-two potentially relevant symptoms in iMCD were identified. Draft frequency and impact questions were developed for each symptom (**Figure 2**). A recall period of the past week and 4-point response scales were used based on patient and clinical advice. Preliminary findings from cognitive interviews (n=10) suggest the draft PROM was comprehensive. Most symptoms were generally well-understood, with only minor revisions (e.g., “unintentional weight loss” vs. “weight loss”). Overlap between some symptoms was recognised with participants having preferences for certain concepts (e.g., “tiredness” vs. “fatigue”). Most symptoms were considered relevant, with areas of redundancy identified (e.g., “sadness” and “depression” were less commonly experienced, and “hopelessness” was rarely relevant).

The figure shows two examples of draft PROM content. The top example is a questionnaire asking about symptoms of iMCD and their impact during the past week, with a 4-point frequency scale (None of the time, Sometimes, Often, Most or all of the time). The bottom example is a 4-point impact scale (No impact, Slight impact, Moderate impact, Severe impact) for the same symptoms.

This questionnaire is asking about symptoms of idiopathic multicentric Castleman disease (iMCD) and how they have impacted your life during the past week .				
For each symptom below, we would like you to consider <u>how often it applied to you</u> during the past week , and, for the symptoms you experienced, we would like you to consider <u>how much of an impact it had on your life</u> during the past week .				
On this page, please read each symptom below and think about <u>how often it applied to you</u> during the past week . Please select the answer that best applies to you.				
Due to iMCD , during the past week I experienced...				
	None of the time	Sometimes	Often	Most or all of the time
Tiredness (i.e., either physical and/or mental tiredness)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Exhaustion	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Fatigue	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Low energy	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Physical weakness	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Dizziness (including feeling faint, weak, or unsteady)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

On this page, please read each symptom below and think about <u>how much of an impact it had on your life</u> during the past week . Please select the answer that best applies to you.				
This iMCD symptom had the following impact on my life during the past week ...				
	No impact	Slight impact	Moderate impact	Severe impact
Tiredness (i.e., either physical and/or mental tiredness)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Exhaustion	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Fatigue	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Low energy	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Physical weakness	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Dizziness (including feeling faint, weak, or unsteady)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Figure 2. Examples of draft iMCD symptom burden PROM content

Summary: This poster describes an international collaboration of iMCD patients, clinicians, academics, and industry experts to develop a novel symptom burden PROM. Successes include a well-performing draft symptom burden scale received positively by people with iMCD in Stage 2 interviews. Challenges include ongoing recruitment issues in an ultra rare disease and necessary adaptations to standard PROM development protocols as a result.

