PCR135

Symptom Burden and its Impact on Daily Living with Idiopathic Multicentric Castleman's Disease: Development and Exploratory Psychometric Validation of a Bespoke International Patient and Caregiver Survey

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INTRODUCTION

- Idiopathic multicentric Castleman's disease (iMCD) is a rare lymphoproliferative disorder driven by proinflammatory hypercytokinaemia
- To our knowledge, this is the first time a survey investigated the patient's perspective of iMCD including symptom type, frequency experience, and associated impact on everyday life

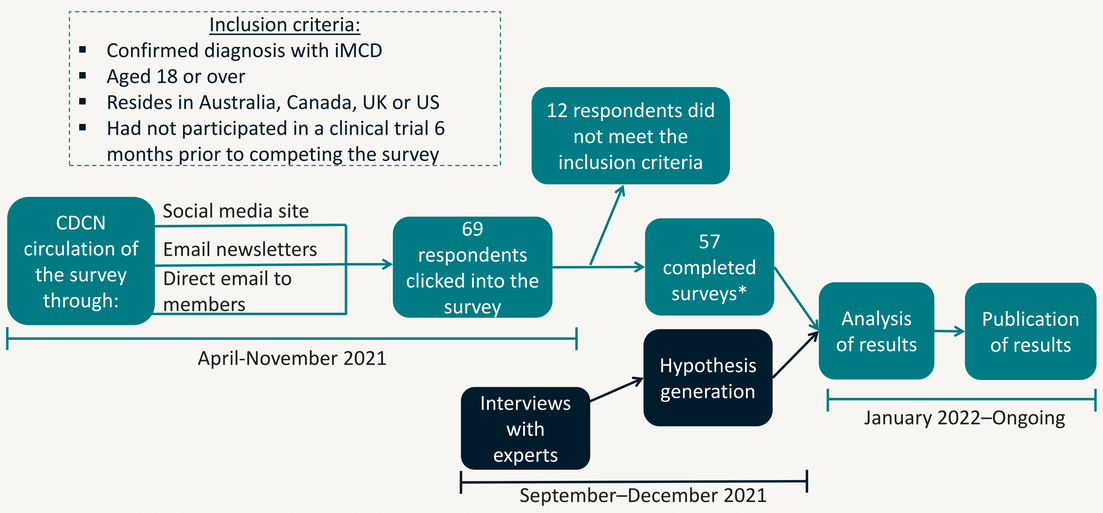
OBJECTIVES

- Our aim was to 1) develop a bespoke international survey to elicit the burden of iMCD and 2) conduct an exploratory analysis of the survey's psychometric properties in terms of internal construct validity (i.e. assessing against factors in the same questionnaire, not against an external questionnaire with a similar construct)

METHODS

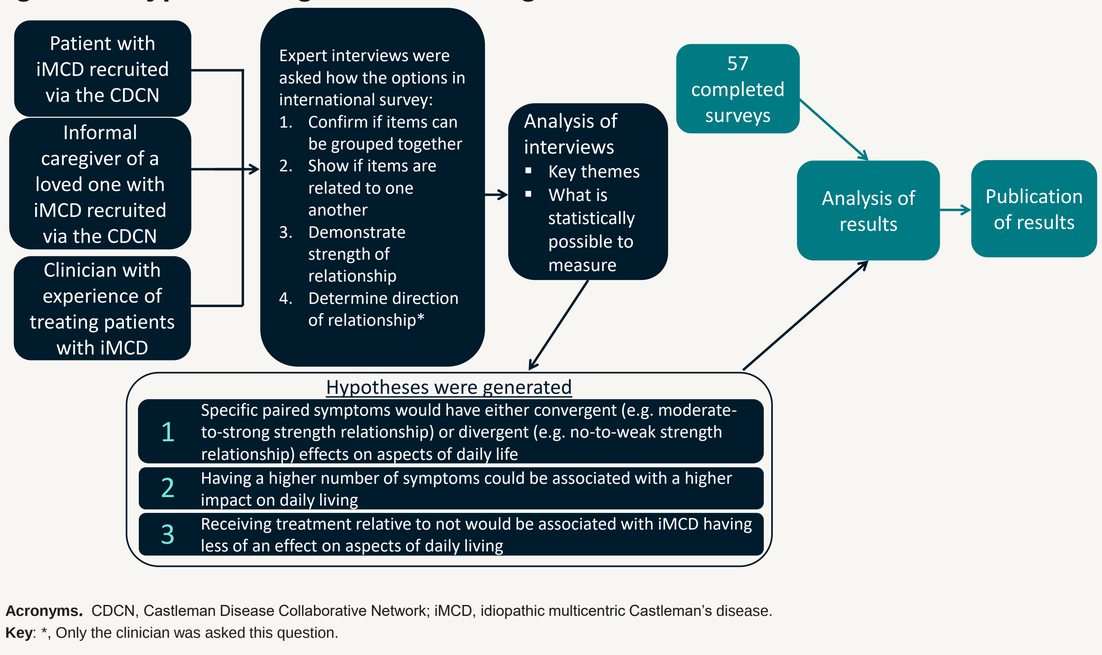
- We developed an international, patient-based, online survey informed by clinical practice and the published literature, taking into account the validated, disease-specific patient-reported outcome measures' language and scaling
- We generated hypotheses based on expert opinion through interviews (one clinician, one patient and one caregiver) to understand the following:
  - Whether specific questions and response options in the survey could be related to one another
  - If items are related, the expected strength of the relationship
  - The potential direction of relationship (for the clinical expert only)
- Spearman's rank absolute correlation strength (ACS) was used to assess the strength and direction of association between two aspects; e.g. for Hypothesis 2, the relationship between the number of symptoms and symptom effect on daily life severity was assessed and hypothesised to be moderate to strong (i.e. ACS > 0.3)
- Figure 1 depicts the overall study programme; Figure 2 provides study design details

Figure 1: Overall study progression



Acronyms. CDCN, Castleman Disease Collaborative Network ; iMCD, idiopathic multicentric Castleman's disease.
Key: * Please note, not all questions were mandatory, and the overall response rate per question may therefore differ.

Figure 2: Hypothesis generation design



Acronyms. CDCN, Castleman Disease Collaborative Network; iMCD, idiopathic multicentric Castleman's disease.
Key: *, Only the clinician was asked this question.

RESULTS

- Overall, 57 patients with iMCD met the inclusion criteria and completed the survey between April and November 2021
- The mean number of symptoms reported by patients was seven (range: 0–22 symptoms)
- Three a priori hypothesis sets were generated, as seen in Figure 2. This poster focuses on symptom burden and its impact on daily life i.e. Hypothesis Set 2
- Hypotheses Set 2 was supported by our analyses with positive, moderate-to-strong and statistically significant correlations estimated; e.g. Table 1 demonstrates that experiencing a higher number of symptoms was associated with the overall symptoms having a worse effect on specific aspects of daily life

DISCUSSION

- These result have potential practical and research implications.
- Practically, this result could support the idea that treatment focussed on iMCD-associated symptoms could improve aspects of patients' daily lives
- In terms of research, these results indicate that this survey has captured the aspects of daily life that are potentially affected by iMCD-associated symptoms
- A limitation is that no 'gold standard' measure exists for external construct validity assessment

Table 1: Convergent validity between number of symptoms and overall symptoms effect on specific aspects of daily living

Aspects of life	N (% N)	Number of symptoms [a], Mean (SD)	Effect severity [b], Mean (SD)	ACS [c], (*> 0.3)	p-value (*< 0.05)	Supports hypothesis?
Pain/discomfort	49 (86.0%)	6.86 (4.71)	2.16 (1.12)	0.53*	< 0.001*	Yes
Mobility	50 (87.7%)	6.96 (4.72)	1.54 (1.11)	0.56*	< 0.001*	Yes
Diet	49 (86.0%)	6.98 (4.76)	1.04 (1.10)	0.59*	< 0.001*	Yes
Sexual functioning	48 (84.2%)	7.17 (4.68)	2.15 (1.47)	0.47*	< 0.001*	Yes
Emotional and psychological wellbeing	50 (87.7%)	6.96 (4.72)	1.66 (1.08)	0.55*	< 0.001*	Yes
Work/education	50 (87.7%)	6.96 (4.72)	1.60 (1.36)	0.39*	0.005*	Yes
Social life	49 (86.0%)	6.98 (4.76)	1.94 (1.45)	0.69*	< 0.001*	Yes
General routine	50 (87.7%)	6.96 (4.72)	1.58 (1.11)	0.67*	< 0.001*	Yes
Personal relationships	50 (87.7%)	6.96 (4.72)	1.58 (1.34)	0.55*	< 0.001*	Yes
Financial wellbeing	50 (87.7%)	6.96 (4.72)	1.54 (1.43)	0.55*	< 0.001*	Yes
Ability to travel	49 (86.0%)	6.98 (4.76)	1.98 (1.18)	0.56*	< 0.001*	Yes
Other	14 (24.6%)	7.07 (5.36)	1.07 (1.44)	0.64*	0.013*	Yes

Acronyms. ACS, absolute correlation strength; p-value, probability value
Key: [a], Number of symptoms is based on the 26 pre-defined symptoms outlined in Question 12: 'Over the past week, what symptoms have you experienced that you attribute to your iMCD?'. The options of 'no symptoms' or 'other' are not included in the number of symptoms estimation.
[b], 'Effect severity' 'overall effect of symptoms on specific aspects of life' based on Question 15: 'How do the symptoms you attribute to your iMCD affect specific aspects of your life?'. Please note that effect severity is based on a Likert scale from 0 (Does not affect my daily life) to 4 (Very severely affects my daily life), so a higher mean number means worse severity on average.
[C], Spearman's rank correlation coefficient strength defined based on Cohen's ACS cut-offs: weak, < 0.3; moderate, 0.3 < 0.5; strong, ≥ 0.5.

CONCLUSIONS

- To our knowledge, this is the first study to characterise symptom burden of iMCD and its impact on daily living
- The exploratory psychometric assessment provides a level of confidence in the construct validity of the survey. These findings could potentially be used to develop a symptom burden score that can help when assessing disease severity, making treatment decisions, and evaluating responses in daily practice and clinical research
- Generating a new condition-specific measure requires initial internal assessment before further development and validation
- This exploratory analyses also highlighted the challenges of working with naturally small sample sizes due to the rare nature of the condition, and the importance of recognising the limitations of what can be achieved with such a data set, given the data collection design

An electronic version of the poster can be viewed by scanning the QR code.

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