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904.OUTCOMES RESEARCH-NON-MALIGNANT CONDITIONS

Symptom Burden in Idiopathic Multicentric Castleman Disease: Protocol for the Development of a Novel Standardized Patient Reported Outcome Measure

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Background and Significance

Idiopathic multicentric Castleman disease (iMCD) is a rare lymphoproliferative disorder with a high symptom burden. These symptoms impact many aspects of daily life for people living with iMCD, including work/education, social life, travel, mobility, personal relationships and sexual functioning. Despite this significant impact [Shupo F et al. Hemasphere 2022, Jun 23;6(Suppl):802-803], no validated disease-specific measure exists to assess symptom burden in iMCD. This is a major unmet need, considering the mainstay of iMCD treatment is symptom control and preventing serious complications. A novel symptom burden scale for iMCD is therefore timely and important and would be a beneficial clinical tool, both in routine clinical practice and clinical trials, for the comprehensive assessment of disease burden at diagnosis, during progression, and for treatment efficacy.

Study Design and Methods

A protocol for the development of a novel, standardized, patient reported outcome measure (PROM) for assessing symptom burden in iMCD has been developed and approved by a multi-stakeholder group (including patients, clinicians, industry representatives, and researchers). A four-stage development process has been proposed, alongside the generation of a novel patient advisory group and wider multi-stakeholder advisory group.

In stage one, draft PROM content will be generated from existing literature and expert opinions. In stage two, the content validity of the draft PROM will be assessed in online qualitative interviews with people living with iMCD, with any revisions to the content decided in consultation with patient and expert advisors. In stage three, the revised PROM will be administered alongside existing measures of symptom burden and/or health-related quality of life to evaluate its psychometric performance and inform decisions on content for the final PROM, using classical test theory and/or Rasch psychometric analyses. The PROM will be finalized based on the qualitative and quantitative evidence generated in consultation with project advisors. Finally, in stage four, the PROM will be re-administered to observe change in symptom burden over time. This will be complemented with qualitative interviews in a mixed methods design to estimate a minimally clinically important difference (MCID) for the measure. The project will involve investigators and iMCD patients from the U.S., U.K., Canada, Australia, New Zealand and Brazil.

Conclusion

This is an international effort to develop and validate a novel iMCD symptom burden PROM through a collaboration of iMCD patients, clinical, health economics, public health, statistical, psychometrics, and industry experts. The development of the measure follows U.S. Food and Drug Administration (FDA) regulatory guidance, with modifications for rare diseases as required.

Special Considerations

This project has been submitted to clintrials.gov, documentation sent to ASH and Secretary, and awaiting approval of the submission.

Disclosures Mukherjee: *McGraw Hill Hematology Oncology Board Review:* Honoraria; *Bristol Myers Squibb:* Honoraria; *EUSA:* Other: Advisory Board; *Aplastic Anemia and MDS International Foundation:* Honoraria; *Celgene (now BMS):* Honoraria; *Genentech and AbbVie:* Other: Advisory Board; *Blueprint Medicines Corporation:* Other: Advisory Board; *Novartis:* Other: Advisory Board; *Bristol Myers Squibb:* Other: Advisory Board; *Celgene/Acceleron:* Other: Advisory Board; *EUSA:* Honoraria; *Celgene (now BMS):* Consultancy; *BioPharm:* Consultancy; *Celgene (now BMS):* Research Funding; *Novartis:* Consultancy; *Bristol Myers Squibb:* Consultancy; *Novartis:* Research Funding; *Jazz Pharmaceuticals:* Research Funding. **Powell:** *KMC Health Care:* Consultancy. **Carlton:** *KMC Health Care:* Consultancy. **Murukia:** *KMC Health Care:* Consultancy. **Brandão:** *Recordati:* Honoraria. **Ramasamy:** *Takeda:* Honoraria, Membership on an entity's Board of Directors or advisory committees; *Pfizer:* Honoraria, Membership on an entity's Board of Directors or advisory committees; *Amgen:* Honoraria, Membership on an entity's Board of Directors or advisory committees, Research Funding; *Recordati:* Honoraria; *BMS (Celgene):* Honoraria, Membership on an entity's Board of Directors or advisory committees, Research Funding; *Janssen:* Honoraria, Membership on an entity's Board of Directors or advisory committees, Research Funding; *Sanofi:* Honoraria, Membership on an entity's Board of Directors or advisory committees; *Adaptive Biotech:* Honoraria, Membership on an entity's Board of Directors or advisory committees; *GSK:* Honoraria, Membership on an entity's Board of Directors or advisory committees, Research Funding; *Menarini Stemline:* Honoraria, Membership on an entity's Board of Directors or advisory committees. **Kanhai:** *Recordati Pharma Ltd:* Current Employment. **Makarounas-Kirchmann:** *Recordati:* Consultancy. **Keetharuth:** *KMC Health Care:* Consultancy.

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