The OMERACT Core Domain Set for Clinical Trials in Behçet’s Syndrome

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Background: There is an unmet need for reliable, validated, and widely-accepted outcome measures for clinical trials in Behçet’s syndrome (BS). Objectives: The Outcome Measures in Rheumatology Clinical Trials (OMERACT) Behçet’s Syndrome Working Group has worked to advance the creation of a data-driven Core Domain Set for use in all clinical trials.

Methods: The Core Domain Set was developed through a comprehensive, iterative, multi-stage, multi-year project that followed the methodologically rigorous processes and standards set forth by OMERACT: i) a systematic review; ii) a survey among experts in BS; iii) an outcome measures interest group meeting during the International Conference on Behçet’s Disease; iv) qualitative patient interviews; v) a three-round modified Delphi exercise involving both patients with BS and a multidisciplinary set of physicians expert in BS, focused on obtaining consensus on the domains of data necessary in the study of BS; and vi) utilization of the data, insight, and feedback generated by the outlined processes to develop a final Core Domain Set. The final Core Set was presented and put up for a vote of endorsement at the 2018 OMERACT meeting.

Results: All steps in the processes outlined were completed. The systematic review clearly demonstrated the substantial variability in the domains studied in clinical trials of BS and a lack of availability of validated outcome measures in BS. The survey of physicians, the in-person meeting of experts, and the qualitative research with patients all helped generate an extensive list of candidate domains and sub-domains to consider for use in clinical trials. It also become clear that there was a need and strong interest in delineating domains across the several major organ systems involved in this disease and in recognizing that clinical trials in BS often focus on specific manifestations and not the disease in its entirety. The Delphi involved 74 physicians expert in BS from 21 countries and from a wide range of specialties, and 64 patients from 10 countries. The Delphi utilized both rankings and ratings to prioritize the 56 domains and sub-domains originally under consideration.

The final proposed Core Set included 5 sub-domains mandatory for study in all trials in BS, with additional sub-domains mandatory for study of specific organ-systems when that system is the focus of a trial: mucocutaneous (2 additional sub-domains), ocular (4), central nervous system (3), musculoskeletal (2), vascular (4), and gastrointestinal (2). The final Core Set was strongly endorsed at the 2018 OMERACT meeting.

Conclusion: Multiple disease-related domains in BS have been identified by physicians and patients as important to address in clinical trials, leading to the development and endorsement of a final Core Set of Domains for use in clinical trials in BS. The Core Set provides the foundation through which the international research community, including clinical investigators, patients, the biopharmaceutical industry, and government regulatory bodies can harmonize the study of this complex disease, compare findings across studies, and advance development of effective agents.

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