NINDS Common Data Elements for Congenital Muscular Dystrophy Clinical Research: A National Institute for Neurological Disorders and Stroke Project.

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Abstract

BACKGROUND: A Congenital Muscular Dystrophy (CMD) Working Group (WG) consisting of international experts reviewed common data elements (CDEs) previously developed for other neuromuscular diseases (NMDs) and made recommendations for all types of studies on CMD.

OBJECTIVES: To develop a comprehensive set of CDEs, data definitions, case report forms and guidelines for use in CMD clinical research to facilitate interoperability of data collection, as part of the CDE project at the National Institute of Neurological Disorders and Stroke (NINDS).

METHODS: One working group composed of ten experts reviewed existing NINDS CDEs and outcome measures, evaluated the need for new elements, and provided recommendations for CMD clinical research. The recommendations were compiled, internally reviewed by the CMD working group, and posted online for external public comment. The CMD working group and the NIH CDE team reviewed the final version before release.

RESULTS: The NINDS CMD CDEs and supporting documents are publicly available on the NINDS CDE website (https://www.commondataelements.ninds.nih.gov/CMD.aspx#tab=Data_Standards). Content areas include demographics, social status, health history, physical examination, diagnostic tests, and guidelines for a variety of specific outcomes and endpoints. The CMD CDE WG selected these documents from existing versions that were generated by other disease area working groups. Some documents were tailored to maximize their suitability for the CMD field.

CONCLUSIONS: Widespread use of CDEs can facilitate CMD clinical research and trial design, data sharing and retrospective analyses. The CDEs that are most relevant to CMD research are like those generated for other NMDs, and CDE documents tailored to CMD are now available to the public. The existence of a single source for these documents facilitates their use in research studies.
and offers a clear mechanism for the discussion and update of the information as knowledge is gained.

**KEYWORDS:** Common data elements; congenital muscular dystrophy; neuromuscular disease; standardization

PMID: 29480213  DOI: [10.3233/JND-170248](https://doi.org/10.3233/JND-170248)