Effectiveness of pharmacological treatments in Duchenne muscular dystrophy: A protocol for a systematic review and meta-analysis

Morena C.P.

Martinez-Vizcaino V.

Álvarez-Bueno C.

Rodríguez R.F.

López E.J.

Torres-Costoso A.I.

Cavero-Redondo I.

Introduction In recent years, important advances have been made in the treatment of Duchenne muscular dystrophy (DMD). This protocol proposes a methodology for carrying out a systematic review and meta-analysis that aims to: (1) improve the evidence of the benefits of different pharmacological treatments in boys with DMD, and (2) compare the benefit of treatments specifically aimed at delaying the progression of disease in the functional outcomes. Methods and analysis This protocol is guided by the Preferred Reporting Items for Systematic Review and Meta-Analysis Protocols (PRISMA-P) and by the Cochrane Collaboration Handbook. A thorough selection of the literature will be done through the MEDLINE, EMBASE and Web of Science databases. The search will be conducted in English and Spanish. The Risk of Bias 2.0 tool from the Cochrane Collaboration will be used to assess the risk of bias. A narrative synthesis of the data will be performed. Meta-analysis will be conducted for effect of treatment on the 6 min walking distance (6MWD), North Star Ambulatory Assessment and Timed Functional Tests. Subgroup analyses will be performed by age or baseline values of the 6MWD, and overall bias. Ethics and dissemination The approval of an ethical committee is not required. All the included trials will comply with the current ethical standards and the Declaration of Helsinki. The results of this proposed systematic review and meta-analysis will provide a general overview and evidence concerning the effectiveness of pharmacological treatments in Duchenne muscular dystrophy. Findings will be disseminated to academic audiences

through peer-reviewed publications, as well as to clinical audiences, patients' associations and policy makers, and may influence guideline developers in order to improve outcomes for these patients. PROSPERO registration number CRD42018102207. © 2019 Author(s).

Duchenne muscular dystrophy

dystrophinopathy

exon skipping

meta-analysis

nonsense readthrough

protocol

systematic review

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casimersen

drisapersen

eteplirsen

gentamicin

golodirsen

disease exacerbation

drug efficacy

Duchenne muscular dystrophy

exon skipping

functional status assessment

human

male

meta analysis

North Star Ambulatory Assessment

Review

six minute walk test

systematic review

Timed Functional Test