Whether stem cell therapy is effective and safe compared to standard care in muscular dystrophies? A systematic review

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INTRODUCTION

- Muscular dystrophies are heterogeneous disorders resulting in progressive muscular weakness, functional dependency, and early mortality.
- Different forms, routes and doses of stem cell therapies with impact on variable outcomes at different time points in muscular dystrophies, especially DMD .

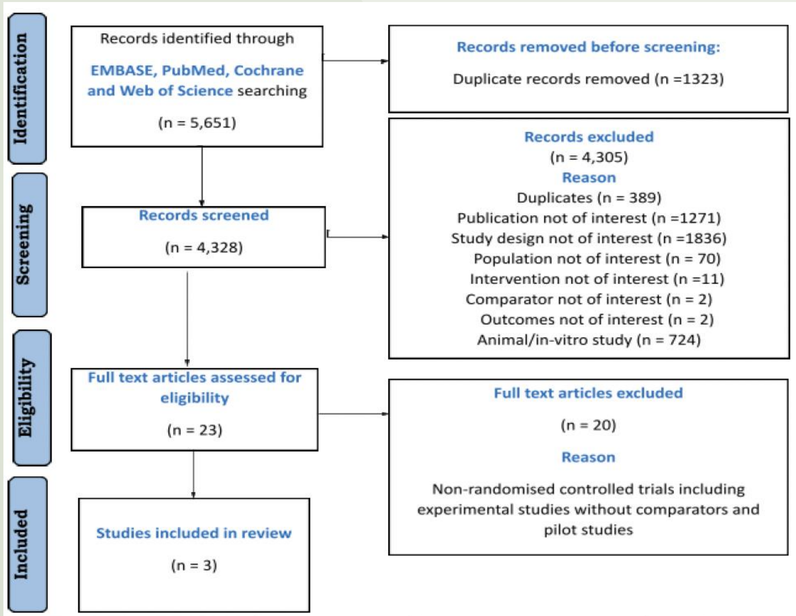

Fig.1 The PRISMA flow diagram of literature search and for selection of studies

MATERIALS & METHODS

- Searched different databases (Web Of Science, PubMed, and EMBASE) using the following major keywords: “Muscular Dystrophy” & "Stem cell therapy".
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- Randomized Controlled Trials (RCTs) comparing the efficacy and safety of stem cell therapy with placebo or usual care in patients with Muscular Dystrophy were included in this review.
- Cochrane risk-of-bias tool for randomized trials was used for quality assessment.

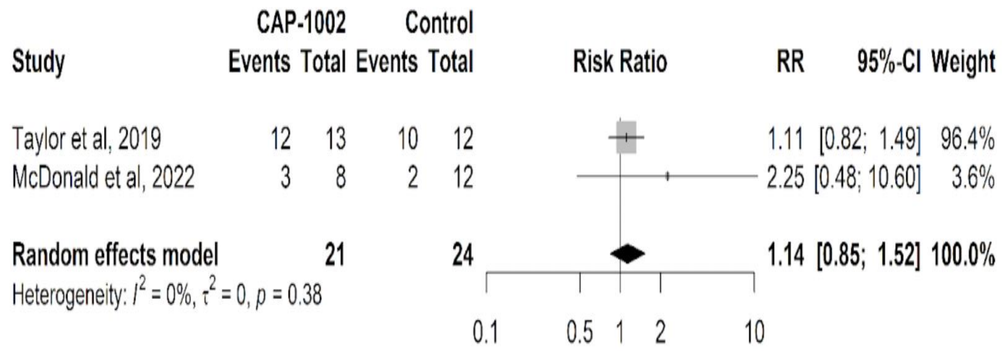

Fig. 2- Pooled estimates of the occurrence of treatment emergent adverse events with CAP-1002 versus Control group

Fig. 3- Risk of Bias Asessment of Included Studies

RESULTS

- Two (HOPE and HOPE-2) trials have shown improved structure with CAP-1002 treatment but this did not translate into improved ejection fraction at 6&12months of treatment.
- Both the trials had shown sustained or improved mid-distal level PUL 1.2 scores with CA-1002 at 6 weeks, 3, 6 and 12 months.
- HOPE trial resulted with approximately similar responses of QoL using patient-and parent-proxy Pediatric Outcomes Data Collection Instrument (PODCI) and PedsQL in both the treatment group.
- Higher numbers of Treatment-emergent adverse events (TEAEs) with the treatment of CAP-1002 (15/21 (71%)) versus control (10/24 (42%)) in patients with DMD.

CONCLUSIONS

- Stem cell therapy may have little to no difference in clinical improvement and quality of life in persons with muscular dystrophies.
- More studies with longer follow ups, larger sample sizes and meaningful outcomes are required to establish potential role of stem cell therapy in muscular dystrophies.

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