**Supplementary materials**

**Supplementary Table 1.** **Search strategy for Ovid MEDLINE® (1 January 2000 to 30 November 2020).**

MEDLINE®

|  |  |
| --- | --- |
| 1. | deflazacort.tw |
| 2. | prednisone.tw |
| 3. | 1 AND 2 |
| 4. | Duchenne muscular dystrophy.tw |
| 5. | DMD.tw |
| 6. | Duchenne.tw |
| 7. | nonsense mutation Duchenne muscular dystrophy.tw |
| 8. | nmDMD.tw |
| 9. | 4 OR 5 OR 6 OR 7 OR 8 |
| 10. | 3 AND 9 |
| 11. | limit 10 to English language |
| 12. | limit 11 to human |
| 13. | limit 12 to “review articles” |
| 14. | 12 NOT 13 |
| 15. | limit 14 to ed="20000101-20201130" |

**Supplementary Table 2. Search strategy for Ovid EMBASE® (1 January 2000 to 30 November 2020).**

EMBASE®

|  |  |
| --- | --- |
| 1. | deflazacort.tw |
| 2. | prednisone.tw |
| 3. | 1 AND 2 |
| 4. | Duchenne muscular dystrophy.tw |
| 5. | DMD.tw |
| 6. | Duchenne.tw |
| 7. | nonsense mutation Duchenne muscular dystrophy.tw |
| 8. | nmDMD.tw |
| 9. | 4 OR 5 OR 6 OR 7 OR 8 |
| 10. | 3 AND 9 |
| 11. | limit 10 to English language |
| 12. | limit 11 to human |
| 13. | limit 12 to (editorial or erratum or letter or note or patent or reports or "conference review" or "review") |
| 14. | 12 NOT 13 |
| 15. | limit 14 to dd="20000101-20201130" |

**Supplementary Table 3.** **Primary and *post-hoc* studies of patients receiving deflazacort or prednisone/prednisolone.**

|  |  |  |  |
| --- | --- | --- | --- |
| **ClinicalTrials.gov identifier** | **Study type** | **Intervention** | **Reference** |
| NCT00592553 | Ataluren Phase 2b trial  | Ataluren vs placebo | Bushby *et al.* 2014 [1] |
| NCT01826487 | Ataluren Phase 3(ACT DMD) trial  | Ataluren vs placebo | McDonald *et al.* 2017 [2] |
| NCT01865084 | Tadalafil Phase 3 trial  | Tadalafil vs placebo | Victor *et al.* 2017 [3] |
| NCT01826487 | *Post-hoc* analysis of placebo arm of ACT DMD trial | Deflazacort vs prednisone/prednisolone | Shieh *et al.* 2018 [4] |
| NCT01865084andNCT01826487 | *Post-hoc* meta-analysis of the placebo arms from the ACT DMD and tadalafil phase 3 trials | Deflazacort vs prednisone/prednisolone | McDonald *et al.* 2020 [5] |
| NCT00468832 | Observational study of DMD patients in the CINRG Duchenne Natural History Study  | Deflazacort vs prednisone/prednisolone | Bello *et al.* 2015 [6] |
| NCT00468832 | Observational study of DMD patients in the CINRG Duchenne Natural History Study | Deflazacort vs prednisone/prednisolone | McDonald *et al.* 2018 [7] |
| NCT01603407 | FOR-DMD Phase 3 trial | Deflazacort vs prednisone | Unpublished [8] |
| Not disclosed | Phase 3 trial of deflazacort and prednisone | Deflazacort vs prednisone vs placebo | Griggs *et al.* 2016 [9] |
| Not disclosed | Observational study of DMD patients from Cincinnati Children’s Hospital Medical Center | Deflazacort vs prednisone/prednisolone | Marden *et al.* 2020 [10] |
| Not disclosed | Retrospective chart review of patients with dystrophinopathies in the USA who were treated withprednisone/prednisolone before being switched to deflazacort | – | Marden *et al.* 2021 [11] |

ACT DMD, Ataluren Confirmatory Trial in Duchenne Muscular Dystrophy; CINRG, Cooperative International Neuromuscular Research Group; DMD, Duchenne muscular dystrophy; FOR-DMD, Finding the Optimum Regimen for Duchenne Muscular Dystrophy.

1. Bushby K, Finkel R, Wong B *et al*. Ataluren treatment of patients with nonsense mutation dystrophinopathy*.* *Muscle Nerve* 50(4), 477–487 (2014).

2. McDonald CM, Campbell C, Torricelli RE *et al*. Ataluren in patients with nonsense mutation Duchenne muscular dystrophy (ACT DMD): a multicentre, randomised, double-blind, placebo-controlled, phase 3 trial*.* *Lancet* 390(10101), 1489–1498 (2017).

3. Victor RG, Sweeney HL, Finkel R *et al*. A phase 3 randomized placebo-controlled trial of tadalafil for Duchenne muscular dystrophy*.* *Neurology* 89(17), 1811–1820 (2017).

4. Shieh PB, McIntosh J, Jin F *et al*. Deflazacort vs prednisone/prednisolone for maintaining motor function and delaying loss of ambulation: a post hoc analysis from the ACT DMD trial*.* *Muscle Nerve* 58(5), 639–645 (2018).

5. McDonald CM, Sajeev G, Yao Z *et al*. Deflazacort vs prednisone treatment for Duchenne muscular dystrophy: a meta-analysis of disease progression rates in recent multicenter clinical trials*.* *Muscle Nerve* 61(1), 26–35 (2020).

6. Bello L, Gordish-Dressman H, Morgenroth LP *et al*. Prednisone/prednisolone and deflazacort regimens in the CINRG Duchenne Natural History Study*.* *Neurology* 85(12), 1048–1055 (2015).

7. McDonald CM, Henricson EK, Abresch RT *et al*. Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study*.* *Lancet* 391(10119), 451–461 (2018).

8. ClinicalTrials.gov. Finding the Optimum Regimen for Duchenne Muscular Dystrophy (FOR-DMD). Available from: <https://clinicaltrials.gov/ct2/show/NCT01603407>*.* (2021).

9. Griggs RC, Miller JP, Greenberg CR *et al*. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy*.* *Neurology* 87(20), 2123–2131 (2016).

10. Marden JR, Freimark J, Yao Z, Signorovitch J, Tian C, Wong BL. Real-world outcomes of long-term prednisone and deflazacort use in patients with Duchenne muscular dystrophy: experience at a single, large care center*.* *J Comp Eff Res* 9(3), 177-189 (2020).

11. Marden JR, Santos C, Pfister B *et al*. Steroid switching in dystrophinopathy treatment: a US chart review of patient characteristics and clinical outcomes*.* *J. Comp .Eff. Res.* doi:10.2217/cer-2021-0110 (2021).