FULL-LENGTH ARTICLE NPub.org/rc64yq

Vamorolone trial in Duchenne muscular dystrophy shows dose-related improvement of muscle function

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Study objective and summary result

The present study investigated whether vamorolone is an effective treatment for Duchenne muscular dystrophy (DMD), and the results indicated that this is the case.

Classification of evidence

Class IV.

What is known and what this paper adds

Glucocorticoids are currently the standard therapeutic option for patients with DMD, but with a substantial burden of side effects. This study provides tentative evidence for vamorolone's efficacy as a treatment for DMD, with reduced side effects.

Participants and setting

The investigators analyzed data from 48 steroid-naïve boys with DMD (age range, 4 to <7 years) who were enrolled in the VBP15-002 and VBP15-003 trials through sites in the US, Canada, Australia, Israel, Sweden, and the UK between July 2016 and August 2017.

Design, size, and duration

VBP15-002 and VBP15-003 were open-label trials in which the participants received vamorolone suspensions at oral doses of 0.25, 0.75, 2.0, or 6.0 mg/kg/day (12 boys per dose) for 24 weeks. The participants completed the Time to Stand Test (TTSTAND) and other measures of gross motor function at baseline and follow-up timepoints.

Primary outcome measures

The primary outcome was from-baseline TTSTAND time changes at week 24.

Main results and the role of chance

The boys experienced from-baseline TTSTAND time improvements at week 24, with the improvements observed at the 2.0- and 6.0-mg/kg/day doses being greater than those observed at the 0.25-mg/kg/day dose ($p \leq 0.04$).

Harms

Vamorolone was safe and well tolerated at all doses.

Table Dose-specific from-baseline TTSTAND time changes

Vamorolone dose, mg/kg/day	Mean from-baseline TTSTAND time change ± SD, s	*p* Value vs 0.25-mg/kg/day dose
0.25	−0.01 ± 0.066	Reference
0.75	0.00 ± 0.062	0.51
2.0	0.05 ± 0.061	0.02
6.0	0.04 ± 0.045	0.04

Bias, confounding, and other reasons for caution

The groups receiving different doses were not fully matched in terms of ages and weakness levels. Doses were not randomly assigned, and this investigation featured no placebo treatments or blinding.

Generalizability to other populations

This investigation's use of a dose-ranging design over a broad dose range, and its international nature, favors the generalizability of the results.

Study funding/potential competing interests

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Trial registration numbers

NCT02760264 (VBP15-002) and NCT02760277 (VBP15-003) on ClinicalTrials.gov.

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