Duchenne muscular dystrophy (DMD) is a severely debilitating neuromuscular disorder affecting 1 in 3,500–5,000 newborn boys. The condition is characterized by progressive muscle weakness, loss of ambulation, and dying in early adulthood. It is caused by deletions or mutations in the DMD gene, which encodes for the protein dystrophin. The dystrophin protein is crucial for maintaining muscle cell integrity and regulates calcium homeostasis, which is essential for muscle function.

**Background**

- Duchenne muscular dystrophy (DMD) is a sex-linked, autosomal recessive disorder affecting 1 in 3,000–5,000 newborn boys.
- It is caused by mutations in the DMD gene, which encodes for the protein dystrophin.
- Dystrophin deficiency results in muscle weakness, loss of ambulation, and death in early adulthood.
- Drisapersen is a dystrophin read-through therapy for DMD.
- The study aimed to evaluate the long-term efficacy and safety of drisapersen in DMD.

**Methods**

- This was a randomized, double-blind, placebo-controlled clinical trial.
- Participants were 4, 13–18 years old, ambulant DMD males with documented dystrophin deficiency.
- Participants were randomized to receive drisapersen 3 or 6 mg/kg/wk or placebo subcutaneously for 24 weeks.

**Results**

- At Week 24, a clinically meaningful treatment benefit (27.1 m) over placebo in change from baseline in 6-minute walking distance (6MWD) was observed in the drisapersen 6 mg/kg group.
- The treatment benefit was maintained for 24 weeks post-treatment in the drisapersen 6 mg/kg group.
- No statistically significant changes were observed in the drisapersen 3 mg/kg group.

**Safety**

- The incidence of adverse events did not increase over time and was generally consistent with those expected in DMD.
- No serious adverse events were reported.

**Conclusions**

- Drisapersen demonstrated a clinically meaningful treatment difference of 27.1 m on 6MWD at Week 24 over placebo.
- The treatment benefit was maintained in the 24-week post-treatment phase.
- Drisapersen showed a favorable safety profile.

- This study suggests that drisapersen may improve ambulatory function and delay the onset of permanent loss of ambulation in DMD patients.

**References**


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