Idebenone as a novel, therapeutic approach for Duchenne muscular dystrophy: Results from a 12 month, double-blind, randomized placebo-controlled trial

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Abstract

Early mortality in Duchenne muscular dystrophy (DMD) is related to cardiac and respiratory complications. A phase IIa double-blind randomized placebo-controlled clinical trial was conducted to investigate the tolerability and efficacy of idebenone therapy in children with DMD. Twenty-one DMD patients (aged 8\textsuperscript{–}16\textsuperscript{ years}) were randomly assigned to daily treatment with 450\textsuperscript{mg} idebenone (Catena\textsuperscript{®}) (\textit{n} = 13) or placebo (\textit{n} = 8) for 12\textsuperscript{ months}. All subjects completed the study and idebenone was safe and well tolerated. Idebenone treatment resulted in a trend (\textit{p} = 0.067) to increase peak systolic radial strain in the left ventricular inferolateral wall, the region of the heart that is...
earliest and most severely affected in DMD. A significant respiratory treatment effect on peak expiratory flow was observed ($p = 0.039$ for PEF and $p = 0.042$ for PEF percent predicted). Limitations of this study were the small sample size, and a skewed age distribution between treatment groups. Data from this study provided the basis for the planning of a confirmatory study.

Keywords
Duchenne; Muscular dystrophy; Treatment; Idebenone; Cardiac; Cardiomyopathy; Respiratory function

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