Experience with Edasalonexent Demonstrates Ability of 4 to 7 Year-Old Boys with Duchenne Muscular Dystrophy to Take Softgel Capsules in Clinical Trials

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Background

Availability of drugs to treat muscular dystrophies is improving medication availability in children. toddlers and preschoolers with Duchenne muscular dystrophy (DMD) often need to take oral medications containing either large or difficult-to-swallow capsules. Capsule size reduction and swallowing ability are essential to patient adherence and clinical trial feasibility in young participants. Catabasis Pharmaceuticals’ Edasalonexent (EDO51), a small molecule inhibitor of neutrophil elastase, is being evaluated in DMD patients as a potential therapeutic in a randomized, double-blind, placebo-controlled Phase 3 clinical trial (MoveDMD). The age of participants in previous clinical trials for DMD is 12 years or older. Information on capsule swallowing ability is limited in children with Duchenne muscular dystrophy. Clinical trial feasibility in young participants is uncertain.

Methods

Study Design

Capsule size selection and ability to swallow assessed in clinical trials. Clinical trials involved boys with DMD ages 5 to 12 years old. Boys were randomized to receive either EDO51 or placebo. There was a 3:1 ratio of EDO51 to placebo for Phase 2 and a 2:1 ratio for Phase 3. Boys were randomized to one of two 4:4 capsule groups or two of four 3:3 capsule groups at the time of enrollment. At the first assessment of swallowing, boys were randomized to one of two 4:4 capsule groups or two of four 3:3 capsule groups. The phase 3 trial included 12-month randomized controlled trials in two different settings (PolarisDMD and MoveDMD). The PolarisDMD Phase 3 study design included boys ages 5 to 12 years old enrolled in a single institutional setting. The MoveDMD Phase 3 study design included boys ages 5 to 12 years old enrolled in a multi-institutional setting.

Results

Capsule Swallowing Ability at Screening

Results—Capsule Selection by Age and Geography

Softgel Capsules Sizes Were Not a Barrier to Enrollment in Young Boys

Conclusion

Summary of Experience with Edasalonexent Capsules in Catabasis Clinical Trials

Patients and families

- Patient groups
  - PolarisDMD Phase 3 Site Staff
  - MoveDMD Phase 2 Site Staff
  - Catabasis team

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Questions?

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