2. SYNOPSIS

Name of Sponsor/Company: Catabasis Pharmaceuticals, Inc **Name of Finished Product:** CAT-1004 capsules **Name of Active Ingredient:** CAT-1004	Individual Study Table Referring to Part of the Dossier Volume: Page:	*(For National Authority Use Only)*

Title of Study:

An Open-Label Extension Study of Edasalonexent in Pediatric Patients with Duchenne Muscular Dystrophy

Investigators:

30 investigators enrolled at least 1 subject

Study center(s):

30 centers in the United States, Canada, the United Kingdom, Germany, Ireland, Sweden and Australia

Phase of development: 3

Studied period (years):

Date first patient enrolled: 18Mar2019

Date last patient completed: 26Oct2020

The Phase 3 trial, PolarisDMD was terminated early for not meeting the primary endpoint. As a result, activities related to the development of edasalonexent stopped including the CAT-1004-302 Open-Label Study of Edasalonexent in Boys with Duchenne Muscular Dystrophy.

Objectives:

Primary Objective

- To assess the safety and tolerability of long-term treatment of edasalonexent in pediatric patients with Duchenne muscular dystrophy (DMD)

Secondary Objective

- To assess the durability of effects of edasalonexent as measured by North Star Ambulatory Assessment (NSAA), the 10-meter walk/run test (10MWT), time to stand from supine, and the 4-stair climb in pediatric patients with DMD

Number of patients (planned and analyzed):

Planned: 140

Actual: 131

Due to the timing of the termination, analysis was limited to safety. Efficacy analyses were not performed.

Diagnosis and main criteria for inclusion:

- Completion of either CAT-1004-201 or CAT-1004-301 for patients rolling over from CAT-1004-201 or CAT-1004-301.

- For siblings of patients who completed CAT-1004-201 OR CAT-1004-301:
  - A sibling of a patient who completed either CAT-1004-201 OR CAT-1004-301
  - Diagnosis of DMD based on a clinical phenotype with increased serum creatine kinase (CK) and documentation of mutation(s) in the dystrophin gene known to be associated with a DMD phenotype.
  - Male sex by birth
  - Age ≥4.0 to <13.0 years (at the time of consent)

Test product, dose and mode of administration:

Edasalonexent 100 mg/kg/day, administered as approximately 33 mg/kg 3 times per day (TID)

Duration of treatment:

104 weeks

Criteria for evaluation:

Safety:

Safety was evaluated in terms of all treatment-emergent adverse events (TEAEs) and serious adverse events (SAEs), as well as physical examination, growth parameters, vital signs, clinical laboratory parameters (including chemistry, hematology), and adrenal function (adrenocorticotrophic hormone [ACTH] and cortisol levels).

Durability of Effect:

The durability of the effect of edasalonexent was to be assessed by the following endpoints at selected visits:

- Timed function testing (TFT), which includes the 10MWT, 4-stair climb, and stand from supine
- The North Star Ambulatory Assessment (NSAA)

Due to the early termination of the study, efficacy analyses were not performed.

Statistical methods:

The Enrolled Population would have consisted of all patients who sign the informed consent form for this study.

The Safety Population would have consisted of all patients in the Enrolled Population who received at least 1 dose of study drug.

Conclusions:

Due to the limited enrollment duration of most patients in the study, no conclusions could be made. The safety profile of edasalonexent was consistent with previous observations. The most common treatment-related AEs were mild diarrhea. There was one SAE of femur fracture considered unrelated. One patient discontinued due to possibly related AEs of urinary incontinence and intermittent diarrhea. Both were noted as resolved.

A summary of safety can be found in Table 1 and Table 2.

Date of the report:

30Apr2021

Table 1: Overview of Adverse Events in CAT-1004-302

	Edasalonexent 100 mg/kg/day
	n = 115* (n) (%)
Any TEAE	25 (21.7)
Severe TEAE	0 (0.0)
Serious Adverse Events	1 (0.9)
Any drug-related TEAE	11 (9.6)
Discontinuation due to TEAE	1 (0.0)
Deaths	0 (0.0)

* n is approximate based on timing of the data cut

Table 2: Adverse Events by System Organ Class and Preferred Term in CAT-1004-302

	Edasalonexent 100 mg/kg/day
	n = 115* (n) (%)
Gastrointestinal disorders	
Diarrhea	6 (5.2)
Abdominal pain upper	2 (1.7)
Infections and infestations	
Influenza	2 (1.7)
Hordeolum	1 (0.9)
Injury, poisoning and procedural complications	
Fall	2 (1.7)
Femur fracture	1 (0.9)
Metabolism and nutrition disorders	
Decreased appetite	1 (0.9)
Thirst	1 (0.9)
Psychiatric disorders	
Anxiety	1 (0.9)
Dysphemia	1 (0.9)

	Edasalonexent 100 mg/kg/day
	n = 115* (n) (%)
Skin and subcutaneous tissue disorders	
Rash	2 (1.7)
Investigations	
White blood cell decreased	1 (0.9)
Respiratory, thoracic and mediastinal disorders	
Sleep apnea syndrome	1 (0.9)
Surgical and medical procedures	
Endodontic procedure	1 (0.9)
Urinary incontinence	1 (0.9)

Sponsor's Responsible Medical Officer

Joanne M. Donovan, MD, PhD
Chief Medical Officer
Catabasis Pharmaceuticals, Inc.

30 April 2021
Date

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