**Supplementary Material**

**PolarisDMD Study Personnel**

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| Polaris DMD Principle Investigators | Study Coordinators | Clinical Evaluators | Site |
| Partha Ghosh, MD | Quimby Wechter | Amy Pasternak;  Liz Mirek;  Elizabeth Maczek | Boston Children's Hospital, Boston, MA |
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| Crystal Proud, MD | Cara Headrick | Lindsay Schuler | Children’s Hosp. of the King's Daughters, Norfolk, VA |
| Erin Neil Knierbein, DO | Breanna Simpson | Betsy Howell;  Amanda Hughes | University of Michigan, Ann Arbor, MI |
| Han Phan, MD | Tu Tran | Lynette Slovensky;  Cindy Ferrante | Rare Disease Research, Atlanta, GA |
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| Kathy Mathews, MD | Evgenia Folts | Shelley Mockler;  Katie Laubscher | University of Iowa, Iowa City, Iowa |
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| Jonathan McKinnon, MD | Kaitlyn Fell | Katherine Bernardo; Tricia Catalino; Katherine Joines | Las Vegas Clinic, Las Vegas, NV |
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| Jessika Johannsen, MD | Deike Weiss; Jana Eggert-Vockerodt | Inka Nickelsen; Anna-Lena Krumbiegel | University of Hamburg, Hamburg, Germany |
| Talya Dor-Wollman, MD | Osnat Eliav | Elana Simchovitz | Hadassah Medical Center, Jerusalem, Israel |
| Monique Ryan, M med BS, FRACP | Jemima Mitchell | Rachel Kennedy; Kate Carroll; Justine Adams; Katy De Valle | Royal Children's Hospital, Victoria, Australia |
| Manoj Menezes, MD, PhD | Luke Wood | Meghan Harman; Kristy Rose; Jennifer Roberts; Stephanie Duvenage | The Children’s Hospital at Westmead, Westmead, NSW, Australia |

**Table S1: NSAA Sensitivity analyses**

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| --- | --- | --- | --- | --- | --- | --- |
| Population: | Baseline  Edasalonexent | Baseline  Placebo | Change from Baseline  Edasalonexent | Change from Baseline  Placebo | LS Difference: Edasalonexent - Placebo | P-value |
| Full Analysis Set (FAS)  (n=119) | 21.5 | 19.5 | -1.5 | -1.8 | **0.3** | **0.67** |
| Per protocol (n=109) | 21.4 | 19.1 | -1.5 | -1.6 | **0.2** | **0.83** |
| FAS, age ≤ 6.0 (n=77) | 20.2 | 18.9 | 0 | -1.0 | **1.4** | **0.08** |
| FAS, age > 6.0 (n=42) | 23.9 | 20.8 | -4.2 | -3.8 | **-0.2** | **0.89** |
| North America (n=83) | 21.0 | 18.4 | -1.8 | -2.0 | **0.4** | **0.68** |
| Europe/Asia/AUS (n=36) | 22.4 | 22.2 | -0.8 | -1.5 | **0.2** | **0.91** |
| On-site efficacy only  (n=95) | 21.5 | 19.5 | -1.2 | -1.8 | **0.6** | **0.51** |

**Figure S1: Total NSAA scores for the Overall Population and by Age Group for Patients ≤ 6.0 years and > 6.0 years.**



**Full inclusion/exclusion criteria for the Polaris DMD Trial**

For inclusion into the trial, patients were required to fulfill all of the following criteria:

1. Written consent/assent by patient and/or legal guardian as per regional and/or IRB/IEC requirements
2. Diagnosis of DMD based on a clinical phenotype with increased serum creatine kinase and documentation of mutation(s) in the dystrophin gene known to be associated with a DMD phenotype
3. Male sex by birth
4. Age ≥4.0 to <8.0 years (at the time of consent)
5. Able to perform stand from supine without assistance in ≤10 seconds
6. Able to perform the 10MRWT and 4-stair climb
7. Able to swallow placebo capsules at the Screening Visit
8. Followed by a doctor or medical professional who coordinates Duchenne care on a regular basis and willingness to disclose patient's study participation with medical professionals

Any of the following was regarded as a criterion for exclusion from the trial:

1. Use of corticosteroids within 24 weeks prior to Day 1; use of inhaled, intranasal, and topical corticosteroids was permitted
2. Use of an investigational drug, idebenone, or dystrophin-focused therapy within 4 weeks or a period of 5 half-lives duration prior to Day 1 (whichever was longer) or ongoing participation in any other therapeutic clinical trial. *Exception: Patients who had received at least 24 weeks of a stable dose of eteplirsen prior to Day 1, and expected to continue treatment, were eligible.*
3. Use of the following within 4 weeks prior to Day 1: immunosuppressive therapy, warfarin, phenytoin, S mephenytoin, cyclosporine, dihydroergotamine, ergotamine, fentanyl, alfentanil, pimozide, quinidine, sirolimus, tacrolimus, or paclitaxel
4. Use of human growth hormone within 3 months prior to Day 1
5. Documented positive hepatitis B surface antigen, hepatitis C antibody, or human immunodeficiency virus (HIV) or a known risk factor for hepatitis such as a blood transfusion within 12 weeks prior to Day 1
6. Hemoglobin <10.5 g/dL
7. Abnormal gamma-glutamyl transferase (>laboratory’s upper limit of normal)
8. Other prior or ongoing medical condition, known hypersensitivity to omega 3 fatty acids, physical findings, ECG findings, or laboratory abnormality (including but not limited to renal insufficiency or impaired hepatic function) that, in the Investigator’s opinion, could have adversely affected the safety of the patient, made it unlikely that the course of treatment or follow up would be completed, or impaired the assessment of study results (e.g., a gastrointestinal condition that would have impaired fat absorption)
9. In the Investigator’s opinion, unwilling or unable for any reason (e.g., attentional or behavioral issues) to complete all study assessments and laboratory tests and comply with scheduled visits, administration of drug, and all other study procedures