



RARE
DISEASE
RESEARCH

1730 Northeast Expressway, NE
Atlanta, GA 30329
Phone 470-665-3051
Fax 678-550-8877

NOTE TO FILE (NTF)

Date: 22 Apr 2024

Study Title: An Open-Label Study to Evaluate the Safety of Golodirsen in Non-Ambulant Patients with Duchenne Muscular Dystrophy

Protocol ID: 19-06-001

ClinicalTrials.gov ID: NCT04708314

Study Sponsor: Rare Disease Research, LLC in collaboration with Sarepta Therapeutics, Inc.

Subject: Study Results and Statistical Analysis

This is to confirm that there was no statistical analysis completed for the above-mentioned study. The study commenced on 31 Oct 2020 and ended on 13 May 2021 as the sponsor decided to withdraw the IND application (IND 151389) from the FDA to focus their resources on other projects.

Trang Vo, PharmD
Director of Regulatory Affairs

22 Apr 2024

Date (DD/MMM/YYYY)

Han Phan, MD
Principal Investigator

22 APR 2024

Date (DD/MMM/YYYY)