SAREPTA
THERAPEUTICS

Date: 1/15/20

To: Office of Attorney General

AGO.highcostprescriptiondrugs@vermont.gov

From: Sarepta Therapeutics

Re: New Drug Report, Pursuant to 18 V.S.A. § 4637

To Whom It May Concern:

On December 12, 2019, Sarepta Therapeutics notified the Office of the Vermont Attorney General that it introduced VYONDYS 53 into the commercial market at a wholesale acquisition cost (WAC) that exceeds the threshold set for a specialty drug under the Medicare Part D Program. Sarepta Therapeutics hereby provides the following additional information required by 18 V.S.A. § 4637(c) for VYONDYS 53.

(1) A description of the marketing and pricing plans used in the launch of the new drug in the United States and internationally:

Pursuant to 18 V.S.A. § 4637(d), a manufacturer may limit the information reported to that which is otherwise in the public domain or publicly available. Sarepta's marketing and pricing plans for VYONDYS 53 are neither in the public domain nor publicly available.

(2) The estimated volume of patients who may be prescribed the drug:

VYONDYS 53 is an antisense oligonucleotide from Sarepta's phosphorodiamidate morpholino oligomer platform, indicated for the treatment of Duchenne muscular dystrophy in patients with a confirmed mutation amenable to exon 53 skipping. Duchenne is a fatal genetic neuromuscular disorder affecting an estimated one in approximately every 3,500 - 5,000 males born worldwide. (Source: National Institutes of Health, Genetics Home Reference, Duchenne and Becker muscular dystrophy, available at https://ghr.nlm.nih.gov/condition/duchenne-and-becker-muscular-dystrophy; accessed December 2019.) Patients with a 53 mutation represent 8 percent of those with Duchenne. (Source: Aartsma-Rus A, Fokkema I, Verschuuren J, et al. Hum Mutat. 2009;30:293-299.)

(3) Whether the drug was granted breakthrough therapy designation or priority review by the FDA prior to final approval:

VYONDYS 53 was granted priority review by the FDA prior to final approval. It was not granted breakthrough therapy designation.

(4) The date and price of acquisition if the drug was not developed by the manufacturer:

Acquisition date and acquisition price not applicable; Sarepta developed this product.