Vermont New Drug Report (emailed to AGO.highcostprescriptiondrugs@vermont.gov)

NDC: 60923022702

Trade Name: AMONDYS 45

Chemical Name: casimersen

1. A description of the marketing and pricing plans used in the launch of the new drug in the United States and Internationally
    a. Consistent with 18 V.S.A. § 4637, Sarepta is limiting its response to that which is otherwise in the public domain or publicly available. Sarepta's marketing and pricing plans for AMONDYS 45 are neither in the public domain nor publicly available.
2. The estimated volume of patients who may be prescribed the drug
    a. AMONDYS 45 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 45 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 45 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
    a. Yes to Both
4. The date and price of acquisition if the drug was not developed by the manufacturer
    a. N/A