**Patient Appeal Letter VILTEPSO – Non-ambulatory**

Insurance Company Name

Insurance Company Address

Insurance Company City/State/Zip

Re: Request for reconsideration of coverage denial.

Your Name

Type of Insurance

Group/Policy Numbers

Subscriber ID Number

Dear [name of representative] or Claims Review Department,

After consulting with my physician, [doctor’s name], I am formally submitting an appeal of your decision to deny coverage of [his/her] recommended treatment plan for VILTEPSO.

Your letter dated [date of letter] stated that “[quote the exact reasons for denial from the letter]”.

On [date], I/my son/daughter was diagnosed with Duchenne muscular dystrophy. Duchenne muscular dystrophy is caused by mutations in the dystrophin gene. This gene is an x-linked genetic disorder characterized by the progressive loss of skeletal muscle and degeneration, leading to premature death. The primary symptoms of Duchenne muscular dystrophy are caused by a lack of dystrophin in the muscle. Children with Duchenne lose the ability to walk independently and most become reliant on wheelchairs for mobility by their early teenage years. Most individuals with Duchenne experience serious respiratory, orthopedic, and cardiac complications. By the age of 18, the majority of patients require ventilation support at night. The average life expectancy is approximately 25-30 years of age, with respiratory complications and cardiomyopathy being common causes of death. There is no escape from this catastrophic disease.

VILTEPSO has been granted accelerated approved by the FDA based on an increase of dystrophin that was shown to be statistically significant in clinical studies.1,3,5 VILTEPSO is intended to allow for production of an internally truncated but functional dystrophin protein.2 The determination by FDA is that this increase in dystrophin is reasonable likely to predict clinical benefit in patients.

Since the diagnosis, the only medication primarily used by patients like myself/my son/daughter has been corticosteroids **which do not** treat the underlying cause of the disease, a lack of dystrophin.

I am greatly encouraged that my doctor believes my child is/I am a good candidate for VILTEPSO. This is the first FDA approved treatment for the disease and is intended to allow for production of an internally truncated but functional dystrophin protein. In a collective statement published by the leading Duchenne clinicians in the country from Certified Duchenne Care Centers – these experts recommend insurers work with neuromuscular specialists with expertise in care for patients with dystrophinopathy, as well as patients and families, and prominent advocacy organizations, such as Parent Project Muscular Dystrophy, in developing policies.3

I/my son/daughter is currently non-ambulatory, having lost the ability to walk independently at age \_\_\_\_\_. Studies in patients with Duchenne have demonstrated that treatment with VILTEPSO yielded and increase over baseline in dystrophin, the key missing protein in those diagnosed with Duchenne.2,3,5 The hope is I/my son/daughter can maintain critical arm function and respiratory function by slowing disease progression. In Duchenne, every day represents the loss of precious muscle.

Please read Dr. [name]’s Letter of Medical Necessity, which is included in this packet. In this letter, Dr. [name] describes my medical history, diagnosis and the rationale used in determining that I should have access to VILTEPSO. Delay in treatment means the loss of critical function and a delay of the ability to produce dystrophin for my/my child’s muscles.

Please contact Dr. [name] or me if you need more information about the efficacy, safety and effectiveness of VILTEPSO. For your information, I have attached peer review studies on VILTEPSO.

I look forward to hearing from you. My contact information is listed below.

Sincerely,

Your Name

Your Street Address, E-mail Address, Phone Number, Fax Number, Cell Phone Number

cc: Doctors’ Names

Employer’s Name

Enclosures: [Provide a list of everything in your appeals packet].

Include a Statement of Medical Necessity from your medical provider.

Publications/references:

1 *FDA Approves Targeted Treatment for Rare Duchenne Muscular Dystrophy Mutation*[*https://www.fda.gov/news-events/press-announcements/fda-approves-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation*](https://www.fda.gov/news-events/press-announcements/fda-approves-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation)

*2 Clemens PR, Rao VK, Connolly AM, et al. Safety, Tolerability, and Efficacy of Viltolarsen in Boys With Duchenne Muscular Dystrophy Amenable to Exon 53 Skipping: A Phase 2 Randomized Clinical Trial. JAMA Neurol. 2020;77(8):982–991. doi:10.1001/jamaneurol.2020.1264*

*3Ionita C, Kinnett K, Mathews K. Collective Statement Regarding Patient Access to Approved Therapies from the Center Directors of Parent Project Muscular Dystrophy’s Certified Duchenne Care Centers. PLOS Currents Muscular Dystrophy. 2018 Mar 15 . Edition 1. doi: 10.1371/currents.md.4a12c57a46a24603cb3d36d7fe0668b6.*

*4 Clemens, PR., et al. “Long-Term Functional Efficacy and Safety of Viltolarsen in Patients with Duchenne Muscular Dystrophy.” Journal of Neuromuscular Diseases, vol. 9, no. 4, 2022, pp. 493–501., https://doi.org/10.3233/jnd-220811.*