**Patient Appeal Letter VYONDYS 53 - 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 VYONDYS 53.

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. Individuals with Duchenne typically lose the ability to walk by middle school, and experience serious respiratory, orthopedic, and cardiac complications due to the lack of dystrophin in their muscles.

VYONDYS 53 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 VYONDYS 53 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 VYONDYS 53. 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 ambulatory. We are hopeful that use of VYONDYS 53 will keep them ambulatory for a longer period of time. Prolonged ambulation in Duchenne is directly correlated with improved pulmonary outcomes later in life. Later age at loss of ambulation predicts higher peak forced vital capacity (FVC) and slower rate of decline in absolute FVC and percent FVC.4 Significant levels of FVC impairment are associated with an increased risk of respiratory infections, complications, and mortality in patients with Duchenne.5,6

Additionally, a 2012 study examining the estimated mean per-patient annual cost of illness by ambulatory class in Duchenne showed a nearly-threefold cost increase between the late ambulatory and late non-ambulatory disease stages.7

Finally, in a rigorous patient preference study conducted by Parent Project Muscular Dystrophy, using state of the art quantitative stated preference methods, the study found that patients prioritized the protection of muscle function over all attributes, and were willing to accept risk and burden in order to achieve stopping or slowing progression of disease.8

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 VYONDYS 53. Delay in treatment means the loss of critical function and a delay of the ability to produce dystrophin for my/my child’s muscles. In Duchenne, every day represents the loss of precious muscle.

Please contact Dr. [name] or me if you need more information about the efficacy, safety, and effectiveness of VYONDYS 53.

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 Diane E. Frank, Eugenio Mercuri, Laurent Servais, Francesco Muntoni,et al. on behalf of the SKIP-NMD Study Group; Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy Neurology May 2020

2 Diane E. Frank, Francesco Muntoni, Eugenio Mercuri et al;. Golodirsen Leads to Sarcolemmal Dystrophin Expression in Patients With Genetic Mutations Amenable to Exon 53 SkippingPediatrics August 2019, 144 DOI: <https://doi.org/10.1542/peds.144.2_MeetingAbstract.241>

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 Humbertclaude et al. European J. of Paediatric Neurology 2012

5 Phillips, M. F., Quinlivan, R. C. M., Edwards, R. H. T., & Calverley, P. M. A. (2001). Changes in Spirometry Over Time as a Prognostic Marker in Patients with Duchenne Muscular Dystrophy, 164, 2191–2194.

6 Finder, J. D., Birnkrant, D., Carl, J., Farber, H. J., Gozal, D., Iannaccone, S. T., ... Sterni, L. (2004). Respiratory care of the patient with duchenne muscular dystrophy: ATS consensus statement. American Journal of Respiratory and Critical Care Medicine, 170(4), 456–465.

7 Erik Landfeldt et al. Neurology 2014;83:529-536

8 Peay, H. L., Hollin, I., Fischer, R., & Bridges, J. F. P. (2014). A community-engaged approach to quantifying caregiver preferences for the benefits and risks of emerging therapies for duchenne muscular dystrophy. Clinical Therapeutics, 36(5), 624–637.