**Patient Appeal Letter - 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 EXONDYS 51.

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.

EXONDYS 51 has been granted accelerated approved by the FDA based on an increase of dystrophin that was shown to be statistically significant in clinical studies. 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 EXONDYS 51. 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.

I/my son/daughter is currently ambulatory. Studies in ambulatory patients with Duchenne Studies have shown over long term follow up that eteplirsen-treated patients demonstrated a statistically significant advantage of 151m (p < 0.01) on 6MWT and experienced a lower incidence of loss of ambulation in comparison to matched HC (n = 13) amenable to exon 51 skipping. Pulmonary Function Test (PFT) results remained relatively stable in eteplirsen-treated patients.1 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.2 Significant levels of FVC impairment are associated with an increased risk of respiratory infections, complications, and mortality in patients with Duchenne.3,4

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.5

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 improved or stabilized muscle function. (6)

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 EXONDYS 51. 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 EXONDYS 51. For your information, I have attached peer review studies, and briefing documents submitted to FDA.

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.

Journal or peer literature supporting the service in question

Eteplirsen FDA Briefing Book. Sarepta Therapeutics

<http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/PeripheralandCentralNervousSystemDrugsAdvisoryCommittee/UCM481912.pdf>

Addendum to Briefing book with additional information

<http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/PeripheralandCentralNervousSystemDrugsAdvisoryCommittee/UCM481913.pdf>

**1J**erry R. Mendell, MD,1,2,3 Nathalie Goemans, MD, PhD,4 Linda P. Lowes, PhD,1,3 Lindsay N. Alfano, PT,1,3 Katherine Berry, PT,1,3 James Shao, MS,5 Edward M. Kaye, MD,5 and Eugenio Mercuri, MD, PhD,6 for the Eteplirsen Study Group and Telethon Foundation DMD Italian Network

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5064753/>

2 Humbertclaude et al. European J. of Paediatric Neurology 2012

3 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.

4 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.

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

6 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.