Patient Appeal Letter AMONDYS 45– 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 AMONDYS 45.

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.

AMONDYS 45 has been granted accelerated approved by the FDA based on an increase of dystrophin. AMONDYS 45 is intended to allow for production of an internally truncated but functional dystrophin protein. 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 AMONDYS 45. 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.[1]

I/my son/daughter is currently non-ambulatory, having lost the ability to walk independently at age _____. The goal is that this therapy can slow the rate of progression of disease allowing for maintaining function critical to activities of daily living such as [list activities of daily living important for you/your child]

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.[2]

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 AMONDYS 45. 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 AMONDYS 45.

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.

References

[1]Ionita 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.

[2] 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.