[Date]

[Name of contact person at insurance company]

[Insurance company name]

[Address]

[City, State, Zip]

Re: [Patient’s name]

[Group/policy number]

[Type of coverage]

Skyclarys denial [date of denial]

[Reason for denial]

Dear [name of contact person at insurance company],

It is my understanding that [name of insurance company] denied coverage of Skyclarys (omaveloxolone) for my patient [patient’s name] because of [reason for denial]. I am writing to appeal this denial, as it is not within the label guidelines approved by the U.S. Food and Drug Administration (FDA) for Skyclarys. In addition, there is nothing in the data from the clinical studies to support this decision.

[Patient’s name] has been under my care since [date] for the treatment of Friedreich’s ataxia (FA). FA is a relentlessly progressive disease, depriving subjects of their ability to ambulate, and later use their hands, speech, and, in many people, vision. Until this past year, there was no treatment for FA. Since I began working with [patient’s name], we have taken several steps to manage [his/her] symptoms including [list current medications, supplements, physical/occupational/speech therapists, other specialists, etc]. As the first and only approved treatment for FA, access to Skyclarys will help improve [patient’s name]’s health outcomes by slowing progression of neurological symptoms.

For this reason, I am writing to you to provide information about Skyclarys. Skyclarys targets a specific cellular dysfunction caused by frataxin deficiency in FA and has been demonstrated in clinical trials (MOXIe Part 1, Part 2 and Open Label Extension) to improve neurological clinical outcomes and activities of daily living. This is why the FDA approved Skyclarys in February 2023, a monumental milestone in the FA community. The trials showed the following results:

* Skyclarys was shown to be generally safe and well-tolerated (MOXIe Part 1 and 2 and Open Label Extension) with few discontinuations or serious adverse events.
* MOXIe Part 2, a randomized, placebo-controlled, double-blind, parallel-group study, met its primary endpoint: statistically significant (p=0.014) improvement from baseline on the modified Friedreich Ataxia Rating Scale (mFARS). The mFARS is a neurological rating scale that is validated in FA to measure upper and lower limb function, balance, gait and speech (all symptoms important and relevant to all individuals with FA). Individuals receiving treatment in the trial demonstrated better scores/benefit across all domains.
* At the end of the MOXIe Part 2 study individuals were enrolled in an Open Label Extension (OLE) study and followed for several years which has provided additional data on the long term safety and efficacy of Skyclarys. In a propensity-matched analysis patients in the MOXIe OLE study were compared to matched-external controls from the Friedreich’s ataxia Clinical Outcome Measures (FA-COMS) natural history study. mFARS scores of untreated patients/controls showed that ***patients in the matched FA-COMS group progressed 6.61 mFARS points at Year 3 versus 3.00 mFARS points for patients in the MOXIe OLE (p<0.0001). Thus, disease progression as assessed by mFARS was slowed by 55% with Skyclarys treatment, an incredible result in this relentlessly and uniformly progressive condition.***

After reviewing this data, ***the FDA approved Skyclarys for all individuals with genetically confirmed FA over the age of 16***. This specific exclusion based on age was made due to the lack of dosing and safety information in younger individuals. There are no restrictions on neurological function scores, ambulatory status, and presence of pes cavus, in the FDA label and should not be applied to criteria for insurance coverage. The FDA approved Skyclarys for broader use because it was shown to be safe and demonstrated slowing of disease progression, an endpoint which is applicable to all individuals living with FA.

USE THIS PARAGRAPH IF DENIED DUE TO NONAMBULATORY STATUS: [Insurance company] has denied [patient’s name] coverage of Skyclarys due to [his/her] non-ambulatory status which, as already stated, is not recommended as a restriction of coverage on the FDA label. This ruling is additionally inappropriate when considering the fact that ***non-ambulatory subjects were included in the MOXIe study*** and had a larger mean improvement in mFARS scores compared to ambulatory subjects. Through the MOXIE studies, Skyclarys was shown to be both safe and effective in non-ambulatory FA patients.

USE THIS PARAGRAPH IF DENIED DUE TO PES CAVUS: [Insurance company] has denied [patient’s name] coverage of Skyclarys due to [patient’s name]’s pes cavus, which, as already stated, is not recommended as a restriction of coverage on the FDA label. This ruling is additionally inappropriate when considering the fact that ***individuals with pes cavus were included in the MOXIe study and showed improvement in activities of daily living*** (Lynch et al, 2021). Although the presence of pes cavus was considered a possible confounding variable when measuring mFARS scores during the MOXIe trials, it was ultimately found that this variable did little to affect results, apatients with pes cavus still showed slowed progression of neurological symptoms when compared to control subjects.

I implore you to reconsider your denial of Skyclarys for [patient’s name]. The data support its use in all patients with FA, including those [who are non-ambulatory/with pes cavus/with specific denial reason]. Should the denial persist, we will appeal to higher civil and legislative authorities.

Please let me know if I can provide further information.

Sincerely,

[Name and credentials]

**References**

Lynch, D. R., Chin, M. P., Delatycki, M. B., Subramony, S. H., Corti, M., Hoyle, J. C., Boesch, S., Nachbauer, W., Mariotti, C., Mathews, K. D., Giunti, P., Wilmot, G., Zesiewicz, T., Perlman, S., Goldsberry, A., O'Grady, M., & Meyer, C. J. (2021). Safety and Efficacy of Omaveloxolone in Friedreich Ataxia (MOXIe Study). *Annals of neurology*, *89*(2), 212–225. <https://doi.org/10.1002/ana.25934>

Lynch, D. R., Chin, M. P., Boesch, S., Delatycki, M. B., Giunti, P., Goldsberry, A., Hoyle, J. C., Mariotti, C., Mathews, K. D., Nachbauer, W., O'Grady, M., Perlman, S., Subramony, S. H., Wilmot, G., Zesiewicz, T., & Meyer, C. J. (2023). Efficacy of Omaveloxolone in Friedreich's Ataxia: Delayed-Start Analysis of the MOXIe Extension. *Movement disorders : official journal of the Movement Disorder Society*, *38*(2), 313–320. <https://doi.org/10.1002/mds.29286>

Lynch, D. R., Goldsberry, A., Rummey, C., Farmer, J., Boesch, S., Delatycki, M. B., Giunti, P., Hoyle, C., Mariotti, C., Mathews, K. D., Nachbauer, W., Perlman, S., Subramony, S. H., Wilmot, G., Zesiewicz, T., Weissfeld, L., Meyer, C. (2022). Direct Utility of Natural History Data in Analysis of Clinical Trials: Propensity Match-based Analysis of Omaveloxolone in Friedreich Ataxia Using the FA-COMS Dataset. MedRxiv. Preprint. <https://www.medrxiv.org/content/10.1101/2022.08.12.22278684v1>

Lynch, D. R., Farmer, J., Hauser, L., Blair, I. A., Wang, Q. Q., Mesaros, C., Snyder, N., Boesch, S., Chin, M., Delatycki, M. B., Giunti, P., Goldsberry, A., Hoyle, C., McBride, M. G., Nachbauer, W., O'Grady, M., Perlman, S., Subramony, S. H., Wilmot, G. R., Zesiewicz, T., … Meyer, C. (2018). Safety, pharmacodynamics, and potential benefit of omaveloxolone in Friedreich ataxia. *Annals of clinical and translational neurology*, *6*(1), 15–26. <https://doi.org/10.1002/acn3.660>