Nevada Medicaid Drug Use Review Board:

SKYCLARYS™ (omaveloxolone) 50 mg capsules is the first and only FDA approved therapy indicated for the treatment of Friedreich ataxia (FA) in adults and adolescents aged 16 years and older.[1] We kindly ask that you make SKYCLARYS accessible to FA patients for the following reasons:

Friedreich Ataxia (FA) Disease Background

FA is a rare, inherited, progressive, neurodegenerative form of ataxia, with average age of onset between 10 – 15 years.[2] It is estimated that there are 5,000 diagnosed patients in the US.[2-5] Patients experience progressive loss of coordination, muscle weakness and fatigue which leads to loss of ambulation, impaired speech, wheelchair reliance in early adulthood, and ultimately leads to severe disability and premature death.[2,3] Being a complex multisystem disorder, FA can also lead to cardiomyopathy, scoliosis, diabetes, hearing loss and optic neuropathy.[2] Management is primarily supportive and typical treatment approaches rely on rehabilitative services.[6] FA is a debilitating disease with a significant impact on quality of life for both patients and families.[4,7] And, US healthcare costs for patients with FA are higher than those of "adults with greater than 2 chronic conditions", mainly driven by homecare and residence costs for care facilities.[8,9]

Diagnosis

Being a genetic movement disorder, FA is diagnosed via clinical evaluation and genetic testing.[10] And, being a rare disorder, it may be difficult for patients to access a Center of Excellence, specialist or neurologist.[11] Also, according to the International Rare Diseases Research Consortium, access to diagnosis and therapies can influence the management and progression of diseases, which can impact not only patients and their families, but also health care systems.[13]

FA Practice Guidelines

For treatment guidelines, the 2022 consensus clinical management guidelines for Friedreich ataxia focuses on supportive care and treatment of individual symptoms associated with FA given that there was no FDA-approved therapy at the time of guideline publication.[11]

SKYCLARYS (omaveloxolone)

SKYCLARYS was approved in February 2023 and became the first and only FDA approved therapy indicated for the treatment of Friedreich ataxia (FA) in adults and adolescents aged 16 years and older.[1]

SKYCLARYS is an oral capsule with a recommended dosage of 150mg (3 capsules) to be taken on an empty stomach once daily.[1] I refer you to the full prescribing information located at www.skyclarys.com for complete efficacy and safety information.[1] SKYCLARYS has also been shown to activate the Nrf2 antioxidant pathway in animals and humans.[1] The Nrf2 pathway is involved in the cellular response to oxidative stress and inflammation.[1]

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The efficacy of SKYCLARYS was established in a 48-week, randomized, double-blind, placebo-controlled trial.[1] FA patients treated with SKYCLARYS achieved a statistically significant improvement over placebo in the validated modified Friedreich Ataxia Rating Scale (mFARS).[1,12] The mFARS is a clinical assessment tool that assesses patient neurological function including bulbar function, upper limb coordination, lower limb coordination and upright stability.[1,15] Patients treated with SKYCLARIS had an overall improvement in their mFARS compared to worsening seen in the placebo population.[1] As well, in a post hoc, propensity-matched analysis, designed to assess persistence of treatment effect, lower mFARS scores were observed in patients treated with omaveloxolone after 3 years relative to a matched set of untreated patients from a natural history study.[1]

For safety, the most common adverse events experienced in greater than 20% or more of patients treated with SKYCLARYS were elevated liver enzymes (37%) but no concomitant elevation of bilirubin, headache, (37%), nausea (33%), abdominal pain (29%), fatigue (24%), diarrhea (20%) and musculoskeletal pain (20%).[1,12]

Real World Outcomes

A recent retrospective study* has shown that FA patients demonstrate greater disease burden than matched cohort individuals without FA. The analysis utilizing a commercial data base of 2,348 FA patients resulted in FA patients showing a greater disease burden including loss of ambulation, physical therapy utilization, cardiac disease and healthcare utilization including emergency department and home health settings.[14]

In closing, SKYCLARYS is the only disease specific therapy approved by the FDA for the treatment of Friedreich ataxia. Based on the improvement in mFARS scores observed in the pivotal trial, omaveloxolone may help address some of the unmet need seen in FA patients including symptom management and by slowing the worsening of progressive disease.

Reata Pharmaceuticals respectfully requests that Nevada Medicaid Drug Use Review Board allow FDA-label access to genetically confirmed FA patients over the age of 16, seeing that there are no package insert contraindications, including ambulatory status, required neurological scores, advanced age and non-presence of pes cavus. Providing FDA label access to SKYCLARYS will allow FA patients to obtain the only therapy approved in this progressive, debilitating and life shortening neurogenetic disease. If the panel requires any additional materials or supporting publications, please reach out to the contact information enclosed below. Thank you for your consideration.

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*Analysis of health insurance claims data pulled from a commercially available database conducted by Reata

References

1. Skyclarys. Prescribing information. Reata Pharmaceuticals, Inc; 2023.
2. Delatycki M., Bidichandani, S. Friedreich ataxia – pathogenesis and implications for therapies. Neurobiol Dis. 2019 Dec;132:104606. doi: 10.1016/j.nbd.2019.104606. Epub 2019 Sep 5.
3. Parkinson MH, et al. J Neurochem. 2013;126(Suppl 1):103-117.
4. National Institute of Neurological Disorders and Stroke. National Institutes of Health. Friedreich ataxia. 2023. Accessed February 10, 2023. https://www.ninds.nih.gov/friedreich-ataxia-fact-sheet.
5. Vankan P. J Neurochem. 2013;126(Suppl 1):11-20
6. Lynch DR, et al. J Multidiscip Healthc 2021;14:1645–58
7. Apolloni S, et al. Int J Mol Sci 2022;23(11):6297
8. Buesch K & Zhang R. Curr Med Res Opin 2022; 38(10):1739–1749
9. Polek B, et al. Front Pharmacol 2013;4:66.Holmström, et al. Curr Opin Toxicol 2016;1:80–91
10. Fogel BL, et al. *Lancet Neurol*. 2007;6(3):245-257
11. Corben L, et al. Clinical management guidelines for Friedreich ataxia: best practice in rare diseases. Orphanet J Rare Dos.2022;17:415
12. Lynch D, et al. Ann Neurol 2021
13. Zanello, G., Chan, CH., Pearce, D.A. *et al.* Recommendations from the IRDiRC Working Group on methodologies to assess the impact of diagnoses and therapies on rare disease patients. Orphanet J Rare Dis 17, 181 (2022). https://doi.org/10.1186/s13023-022-02337-2
14. Song C, et al. Disease Burden in Patients with Friedreich's Ataxia (FA) in the US Real World Evidence (RWE) Retrospective Claims Analysis [poster] March 19-22, 2023. Dallas, TX USA
15. Rummey C, et al. Neurol Genet 2019;5(6)371