BlueCross BlueShield
Federal Employee Program

SKYCLARYS
(omaveloxolone)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Skyclarys (omaveloxolone) is indicated for the treatment of Friedreich's ataxia, which is a genetic, progressive, neurodegenerative movement disorder, with a typical age of onset between 10 and 15 years. Friedreich's ataxia is caused by mutations in the FXN gene by autosomal recessive inheritance. Affected individuals often develop slurred speech, characteristic foot deformities, irregular curvature of the spine, and cardiomyopathy. The precise mechanism by which Skyclarys exerts its therapeutic effect in patients with Friedreich's ataxia is unknown but may be due to its activation of Nuclear factor-like 2 (Nrf2) pathway which is involved in the cellular response to oxidative stress (1-2).

Regulatory Status

FDA-approved indication: Skyclarys is indicated for the treatment of Friedreich's ataxia in adults and adolescents aged 16 years and older (1).

Treatment with Skyclarys can cause an elevation in hepatic transaminases (ALT and AST). ALT, AST, and total bilirubin should be monitored prior to initiation of Skyclarys, every month for the first 3 months of treatment, and periodically thereafter. If transaminases increase to levels greater than 5 times the upper limit of normal (ULN), or greater than 3 times the ULN with evidence of liver dysfunction, Skyclarys should be discontinued immediately, and repeat liver function tests as soon as possible (1).

Skyclarys may cause an increase in B-type natriuretic peptide (BNP), a marker of cardiac function. Elevations in BNP may indicate cardiac failure and should prompt an evaluation of cardiac function. Check BNP prior to initiation of Skyclarys. Patients should be monitored for signs and symptoms of fluid overload, peripheral edema, palpitations, and shortness of breath (1).

Skyclarys may also cause increases in LDL and reductions in HDL cholesterol. Lipid parameters should be assessed prior to initiation with Skyclarys and monitored periodically during treatment (1).

The safety and effectiveness of Skyclarys in pediatric patients less than 16 years of age have not

been established (1).

Summary

Skyclarys (omaveloxolone) is indicated for the treatment of Friedreich's ataxia, a genetic neurodegenerative movement disorder. Patients taking Skyclarys should have ALT, AST, bilirubin, BNP, and lipid parameters monitored. The safety and effectiveness of Skyclarys in pediatric patients less than 16 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Skyclarys while maintaining optimal therapeutic outcomes.

References

1. Skyclarys [package insert]. Cambridge, MA: Biogen US Corporation; December 2024.
2. Friedreich's Ataxia: National Organization for Rare Disorders (NORD). March 15, 2023. https://rarediseases.org/rare-diseases/friedreichs-ataxia/