

Omaveloxolone: Adis Evaluation

Key Points

- A semi-synthetic triterpenoid drug that increases antioxidant activity is being developed by Reata Pharmaceuticals, Inc. for the treatment of Friedreich's ataxia
- Received its first approval on 28 Feb 2023 in the USA
- Approved for use in the treatment of Friedreich's ataxia in adults and adolescents aged 16 years and older

Summary

Omaveloxolone (SKYCLARYS™) is an orally active, small molecule semi-synthetic triterpenoid drug that increases antioxidant activity, which is being developed by Reata Pharmaceuticals, Inc. for the treatment of Friedreich's ataxia.

In patients with Friedreich's ataxia, the nuclear factor (erythroid-derived 2)-like 2 (Nrf2) pathway is suppressed, which is associated with oxidative stress, mitochondrial dysfunction and damage to cells, including central and peripheral neurones. The Nrf2 pathway may be activated by omaveloxolone as it blocks the ubiquitination and degradation of Nrf2.

Omaveloxolone was approved in February 2023 in the USA for the treatment of Friedreich's ataxia. This article summarizes the milestones in the development of omaveloxolone leading to this first approval for the treatment of Friedreich's ataxia in adults and adolescents aged 16 years and older.

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