14 December 2023
EMA/CHMP/535984/2023
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Skyclarys

omaveloxolone

On 14 December 2023, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Skyclarys[2], intended for the treatment of Friedreich's ataxia. The Applicant for this medicinal product is Reata Ireland Limited.

Skyclarys will be available as a 50 mg hard capsule. The active substance of Skyclarys is omaveloxolone (ATC code: not yet assigned). Omaveloxolone activates the Nuclear factor (erythroid-derived 2)-like 2 (Nrf2) pathway which is involved in the cellular response to oxidative stress. The precise mechanism by which omaveloxolone exerts its therapeutic effect in patients with Friedreich's ataxia is unknown, but as Nrf2 activity is reduced in patients with Friedreich's ataxia, Nrf2 activators may be involved.

The benefit of Skyclarys is an improvement in the mFARS (modified FA rating scale) score measuring disease progression, as compared with a placebo control in a randomised clinical trial. The most common side effects are increased ALT and AST, decreased weight and appetite, nausea, vomiting, diarrhoea, headache, fatigue, oropharyngeal and back pain, muscle spasms, and influenza.

The full indication is:

> Skyclarys is indicated for the treatment of Friedreich's ataxia in adults and adolescents aged 16 years and older.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

© European Medicines Agency, 2023. Reproduction is authorised provided the source is acknowledged.