

Experiences and perceptions of patients with Friedreich ataxia treated with omaveloxolone (Skyclarys) in France

Cecilia Estrella, Sandra Lechene, Véronique Lattes, Noémie Carpentier, Juliette Dieusaert, Patricia Etienne, Gauthier Bayard, Isabelle Lafargue, Stéphanie Carlin and Stéphan Rouillon.

French Friedreich Ataxia Patients Association (AFAF)

Background and Objectives

Omaveloxolone (Skyclarys®) is available in France for FA patients under an Early Access Program (EAP) granted by the French National Health Authority since November 2023.

The French Friedreich Ataxia Patients Association (AFAF) collected real-life experiences and perceptions from French patients treated with omaveloxolone (omav) for more than 1 year to support the application for the renewal of the EAP.

Methods

FA patients anonymously agreed to answer a questionnaire about their experiences and perceptions regarding balance, coordination, speech, fatigue and their daily life. Responses were collected from June to July 2025.

Results

From a total of 142 FA patients who answered the survey, 119 were on treatment, representing 27% of patients receiving omav in France. Both ambulatory (n=50) and non-ambulatory (n=67) patients noticed an improvement in speech (44% and 67% respectively), balance (29% and 32%), coordination (33% and 36%) and other motor aspects. 78% of those who experienced an improved speech and 82% of those who reported an improved balance, noticed the improvement within the first 6 months of treatment. All declared a decrease in fatigability (35% and 28% respectively). Moreover, considering all aspects, 42%-75% of ambulatory and 23%-74% of non-ambulatory patients reported neither improvement nor degradation. Side effects (headache, digestive problems, nausea, fatigue, etc.) were mostly reported during the first 3 months of treatment. Overall, 56% of patients said that the treatment improves their quality of life, and 30% expressed satisfaction with Skyclarys.

Discussion and Conclusion

These data revealed expected neurological benefits in terms of coordination and balance, but also improvements in major symptoms affecting quality of life, such as reduced fatigue and improved speech. After more than a year of treatment, perceptions described as "neither improvement nor deterioration" of the disease, could be interpreted as a sense of stability, with the disease remaining unchanged.