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The first treatment (Skyclarys) for Friedreich's ataxia will be reimbursed by the National Health Service

Seven medicines (including three orphan drugs), two generics and six therapeutic indication extensions have been approved for reimbursement by the AIFA Board of Directors

At its meeting on 15 December, the AIFA Board of Directors gave the green light to the reimbursement of Skyclarys (omaveloxolone), an orphan drug for the treatment of Friedreich's ataxia.

Friedreich's ataxia is a rare genetic disease that mainly affects the central nervous system and the heart. It begins during childhood or adolescence and its progression often leads to loss of motor autonomy by the third decade of life, while cardiac complications are the leading cause of mortality. It is estimated to affect 1 in every 20,000–50,000 people worldwide. In Italy, the estimated prevalence is approximately 1.07 cases per 100,000 inhabitants.

Skyclarys, the first medicine proven to slow the progression of the disease, was authorised by the European Medicines Agency (EMA) in February 2024 and included by AIFA in July 2024 in the list of medicines distributed under Law No. 648/1996, an early access programme that allows free dispensing to eligible patients, with the cost entirely covered by the National Health Service.
Now, following negotiations with the marketing authorisation holder, the medicine will be available for reimbursement for the treatment of patients aged 16 years and older, upon prescription by centres for the treatment of rare diseases.

Two other orphan drugs have been approved for reimbursement:

- **Hetronifly** (serplulimab), indicated in combination with carboplatin and etoposide for the first-line treatment of adult patients with extensive-stage small cell lung cancer;
- **Winrevair** (sotatercept), indicated, in combination with other therapies, for the treatment of pulmonary arterial hypertension, to improve exercise capacity.

The Board of Directors also authorised the reimbursement of **Recigar** (Citisina), a new molecule indicated for smoking cessation. The use of Recigar allows for a gradual reduction in nicotine dependence and weaning off tobacco smoking without nicotine withdrawal symptoms (such as depressed mood, irritability, anxiety, difficulty concentrating, insomnia, increased appetite). It is recommended for people aged between 18 and 65. The duration of treatment is 25 days.

The following will also be reimbursed by the National Health Service:

- **Hympavzi** (marstacimab), for routine prophylaxis of bleeding episodes in patients with severe haemophilia A or B;
- **Ibuprofene Gen Orph** (ibuprofene), for the treatment of patent ductus arteriosus in premature infants;
- **Kayfanda** (odevixibat), for the treatment of cholestatic pruritus due to Alagille syndrome (ALGS).

Two generics are also eligible for reimbursement now:

- **Mesalazina DOC** (mesalazina), indicated for ulcerative colitis;
- **Oroxelam** (midazolam), indicated for prolonged acute seizures in children and adults.

Among the extensions of therapeutic indications, the monoclonal antibodies **Keytruda** (pembrolizumab) and **Padcev** (enfortumab vedotin), in combination, will be reimbursed for the treatment of urothelial carcinoma.

Keytruda (pembrolizumab), in combination with other medicines, will also be reimbursed for endometrial and cervical cancer.

For endometrial cancer, an extension of the indication for the medicine **Jemperli** (dostarlimab) will also be reimbursed. Finally, **Pirferidone Sandoz** (pirferidone) will be reimbursed for the treatment of idiopathic pulmonary fibrosis