



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Press Office

Press release

European Medicines Agency recommends approval of new medicine for Cushing's disease

New somatostatin analogue recommended to be first approved medical therapy for rare hormonal disorder

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) today recommended approval of a new medicine intended for the treatment of Cushing's disease in patients who cannot have surgery or for whom surgery has not been successful.

Cushing's disease is a very rare, debilitating and life-threatening disease, where a small tumour of the pituitary (an endocrine gland) makes too much of the hormone adrenocorticotrophin (ACTH), which in turn stimulates the adrenal glands to both grow and release excessive amounts of cortisol in the blood. This causes a set of symptoms including weight gain (particularly in the face and neck), easy bruising, excessive growth of coarse hair on the face, weakening of the muscles and bones, and high blood pressure. Cushing's disease is estimated to affect approximately 0.4 in 10,000 people in the European Union. This is equivalent to a total of around 20,000 people.

The first-line therapy for patients with Cushing's disease is surgical removal of the tumour. However, for patients who cannot be cured by surgery there are currently no approved medical treatments. A number of medicines are being used off-label to treat the disease, but the available data on their safety and efficacy is limited.

The recommendation to approve Signifor (pasireotide) for the treatment of Cushing's disease is the first step in making a medicine available to European patients that has been studied in the indication and for which doctors and patients are provided with specific information about its use.

Signifor is a somatostatin analogue. It attaches to somatostatin receptors, which are found in large numbers in tumour cells of the pituitary gland. By attaching to these receptors, Signifor blocks the

* Correction of note 2, the European Commission grants marketing authorisation, does not adopt opinions on medicines.



release of ACTH. This results in the reduction of cortisol levels, helping to relieve the symptoms of Cushing's disease.

In clinical trials, Signifor was shown to reduce the levels of cortisol in the urine by at least 50% in 41% of patients treated with a 900µg dose and in 34% of patients treated with a 600µg dose. In terms of safety, the CHMP noted that Signifor's safety profile is similar to that of other somatostatin analogues, which have been approved for many years in the EU. The product information specifically advises doctors to monitor patients for liver and heart problems. With these precautions in place, the CHMP concluded that the benefits of Signifor outweigh its risks as a second-line treatment for patients with Cushing's disease and recommended its authorisation in the EU.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The Committee's recommendation has now been forwarded to the European Commission for the final marketing authorisation.
3. Pasireotide was designated as an orphan medicine on 8 October 2009.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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