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Zokinvy (*lonafarnib*)

An overview of Zokinvy and why it is authorised in the EU

What is Zokinvy and what is it used for?

Zokinvy is a medicine used to treat patients of 12 months and older who are affected by the following rare diseases in which features resembling aging appear in childhood:

- Hutchinson-Gilford progeria syndrome;
- processing-deficient progeroid laminopathies.

The diseases that Zokinvy is used to treat are rare and Zokinvy was designated as 'orphan medicine' on 14 December 2018 for Hutchinson-Gilford progeria syndrome.

Zokinvy contains the active substance lonafarnib.

How is Zokinvy used?

Zokinvy can only be obtained with a prescription and treatment should be initiated by doctors with experience in the treatment of patients with premature aging or rare metabolic conditions.

Zokinvy is available as capsules to be taken with food twice a day. The starting daily dose (which ranges from 75 to 225 mg) depends on the height and weight of the patient. After 4 months of treatment, the patient may start taking a higher (maintenance) dose.

For more information about using Zokinvy, see the package leaflet or contact your doctor or pharmacist.

How does Zokinvy work?

Patients with Hutchinson-Gilford progeria syndrome and with processing-deficient progeroid laminopathies have an accumulation of abnormal forms of progerin or progerin-like proteins, which causes damage to cells and leads to symptoms of aging early in life. Zokinvy prevents a chemical reaction involved in the formation of these abnormal proteins, thereby helping to improve symptoms of the diseases.

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What benefits of Zokinvy have been shown in studies?

Two main studies have shown that Zokinvy prolongs the life of patients with Hutchinson-Gilford progeria syndrome and with processing-deficient progeroid laminopathies. The studies involved 62 patients who were given Zokinvy. Three years after start of treatment with Zokinvy only, the patients lived between 2.5 months and about half a year longer than the 62 patients who did not participate in the studies and were not given Zokinvy. At the time of the last follow-up (about 11 years after starting treatment), patients given Zokinvy (and possibly additional treatments) lived an average of 4.3 years longer than untreated patients. However, given the limited data available, the extra years lived could be as low as 2.6 years.

What are the risks associated with Zokinvy?

The most common side effects with Zokinvy (which may affect more than 1 in 10 people) are vomiting, diarrhoea, increased levels of liver enzymes, decreased appetite, nausea, abdominal pain, tiredness, weight loss, constipation and upper respiratory tract infection (nose and throat infection).

The most common serious side effects with Zokinvy (which may affect up to 1 in 10 people) are increased levels of liver enzymes, cerebral ischaemia (reduced blood supply to the brain), fever and dehydration.

Why is Zokinvy authorised in the EU?

At the time of the authorisation of Zokinvy there were no other medicines for treating Hutchinson-Gilford progeria syndrome and processing-deficient progeroid laminopathies. The results from the studies of Zokinvy showed that this medicine can prolong the life of patients with these conditions. The most common side effects, such as diarrhoea, nausea, and vomiting, occurred mainly in the first 4 months of treatment and were manageable.

The European Medicines Agency therefore decided that Zokinvy's benefits are greater than its risks and it can be authorised for use in the EU under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Zokinvy due to the rarity of the disease.

Every year, the European Medicines Agency will review any new information that becomes available, and this overview will be updated as necessary.

What information is still awaited for Zokinvy?

Since Zokinvy has been authorised under exceptional circumstances, the company that markets Zokinvy will provide data from a registry of patients treated with the medicine to further evaluate the safety and effectiveness of Zokinvy as well as the quality of life of patients.

What measures are being taken to ensure the safe and effective use of Zokinvy?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Zokinvy have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Zokinvy are continuously monitored. Suspected side effects reported with Zokinvy are carefully evaluated and any necessary action taken to protect patients.

Other information about Zokinvy

Zokinvy received a marketing authorisation valid throughout the EU on 18 July 2022.

Further information on Zokinvy can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/zokinvy

This overview was last updated in 07-2022.