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Public summary of opinion on orphan designation

Melatonin, sorafenib for the treatment of hepatocellular carcinoma

On 23 September 2019 the Committee for Orphan Medicinal Products (COMP) adopted a negative opinion on the orphan designation application for melatonin, sorafenib for the treatment of hepatocellular carcinoma. A negative decision C(2020)929 was issued by the European Commission on 13 February 2020.

The sponsor applied for orphan designation on the basis of the seriousness and the rarity of the condition, as well as an assumption of potential benefit over currently available methods of treatment.

The negative opinion was based on the following reason:

- The sponsor failed to demonstrate that the medicine could be effective in treating hepatocellular carcinoma in experimental models.

Requests for designation as an orphan medicinal product are made for investigational products. Absence of orphan designation does not preclude the development of this product, including its use in clinical trials. A marketing authorisation can still be obtained if quality, safety and efficacy are demonstrated.

For more information:

Contact details of the current sponsor for this orphan designation can be found on the EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

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