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Media and Public Relations

Press release

New medicine for hereditary rare disease

Tegsedi addresses unmet medical need for treatment of hereditary transthyretin amyloidosis

The European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended granting a marketing authorisation in the European Union (EU) for Tegsedi (inotersen), a medicine for treatment of stage 1 or stage 2 polyneuropathy (a condition in which the peripheral nerves are damaged) in patients with hereditary transthyretin amyloidosis (hATTR). This medicine aims to affect the course of the disease and improve quality of life.

In patients with hATTR, a blood protein called transthyretin is defective and breaks easily. The broken protein forms a fibrous substance called amyloid that builds up in the peripheral nervous system and multiple organs, such as the gastrointestinal tract, kidney and heart, where it interferes with their normal functions.

Tegsedi is an 'antisense oligonucleotide', a very short piece of synthetic DNA designed to attach to the genetic material of the cells responsible for producing the transthyretin protein. This is expected to decrease transthyretin production, thereby reducing the formation of amyloids and relieving the symptoms of hATTR.

The effects of Tegsedi were evaluated in a study involving hATTR patients with stage 1 or stage 2 polyneuropathy. The study showed clinically relevant effects on the neurological manifestations of the disease and on patients' quality of life. The efficacy of this treatment in patients with stage 3 polyneuropathy has not yet been demonstrated.

Current therapeutic options for hATTR are liver transplant, treatment with tafamidis and off-label use of a non-steroidal anti-inflammatory drug (NSAID). All of them have considerable limitations for patients with stage 2 and stage 3 polyneuropathy, meaning there is a clear unmet medical need. Therefore, the CHMP considered that Tegsedi is of major interest for public health and agreed to the applicant's request for an accelerated assessment of this medicine.

As hATTR is a rare disease estimated to be diagnosed in approximately three cases per 10 million people in Europe yearly, Tegsedi was designated as an orphan medicine in 2014.

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As always at time of approval, EMA's Committee for Orphan Medicinal Products (COMP) will review the orphan designation to determine whether the information available to date allows maintaining Tegsedi's orphan status and granting this medicine ten years of market exclusivity.

The opinion adopted by the CHMP is an intermediary step on Tegsedi's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. The applicant for Tegsedi is IONIS USA Ltd
2. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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