10 February 2021
EMADOC-628903358-3052

Public summary of opinion on orphan designation

Sparsentan for the treatment of primary IgA nephropathy

On 19 October 2020, orphan designation EU/3/20/2345 was granted by the European Commission to Travere Therapeutics Ireland Limited, Ireland, for sparsentan for the treatment of primary IgA nephropathy.

What is primary IgA nephropathy?

Primary IgA nephropathy is a disease caused by the immune system (the body's natural defences) producing a faulty version of an antibody called immunoglobulin A (IgA), which builds up in clusters of small blood vessels in the kidney, called glomeruli, that filter the blood. This build-up damages the glomeruli, causing leakage of blood and protein into the urine.

Primary IgA nephropathy is a long-term debilitating and life-threatening disease because the kidneys gradually stop working properly and eventually fail, requiring dialysis or a kidney transplant.

What is the estimated number of patients affected by the condition?

At the time of designation, primary IgA nephropathy affected approximately 4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 208,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, no satisfactory methods were authorised for treating primary IgA nephropathy. Patients were treated with ACE inhibitors or angiotensin receptor blockers to lower blood pressure, and with medicines to suppress the immune system, such as corticosteroids, ciclosporin or cyclophosphamide. As the disease worsens, kidney dialysis and kidney transplant may be needed.

*For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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How is this medicine expected to work?

Sparsentan is a substance that blocks the receptors (targets) for two hormones called endothelin and angiotensin II that are involved in the regulation of blood pressure and kidney function. Blocking the action of angiotensin II allows blood vessels to widen, reducing blood pressure, and helps to reduce the amount of water re-absorbed by the kidneys, improving urine production. Endothelin is thought to be involved in the progression of primary immunoglobulin A nephropathy; blocking its action is expected to reduce damage to the kidney. By blocking the effects of both hormones, the medicine is expected to help slow down the progression of symptoms in patients with the condition.

What is the stage of development of this medicine?

The effects of sparsentan have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with sparsentan in patients with primary IgA nephropathy were ongoing.

At the time of submission, sparsentan was not authorised anywhere in the EU for the treatment of primary IgA nephropathy. Orphan designation of sparsentan had been granted in the EU and in the United States for the treatment of another kidney disease, focal segmental glomerulosclerosis.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 10 September 2020, recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

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