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Public summary of opinion on orphan designation

(4-{(2S,4S)-4-ethoxy-1-[(5-methoxy-7-methyl-1*H*-indol-4-yl)methyl]piperidin-2-yl}benzoic acid-hydrogen chloride(1/1)) for the treatment of primary IgA nephropathy

On 19 October 2020, orphan designation EU/3/20/2336 was granted by the European Commission to Novartis Europharm Limited, Ireland, for (4-{(2S,4S)-4-ethoxy-1-[(5-methoxy-7-methyl-1*H*-indol-4-yl)methyl]piperidin-2-yl}benzoic acid-hydrogen chloride(1/1)) (also known as LNP023) 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

*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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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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.

How is this medicine expected to work?

The medicine blocks the action of an enzyme called complement factor B. Factor B is involved in activating proteins in the 'complement system', which is part of the immune system. The complement system plays a role in inflammation of the kidneys in primary IgA nephropathy. By blocking factor B, the medicine is expected to reduce the immune response that causes the symptoms of the disease.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of primary IgA nephropathy or designated as an orphan medicinal product elsewhere for this condition.

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.