

## INVESTOR & MEDIA UPDATE

### **Novartis announces European Medicines Agency (EMA) has granted orphan drug designation for iptacopan (LNP023) in IgA nephropathy (IgAN)**

- *Orphan drug designation is reserved for medicines treating rare, life-threatening or chronically debilitating diseases*
- *IgA nephropathy (IgAN), while rare, is the most common form of glomerulonephritis, affecting mostly young adults with no approved treatment option and significant risk to progress to end stage renal disease (ESRD) <sup>1,2,3</sup>*
- *Iptacopan (LNP023) is a potential first-in-class, oral, potent and selective factor B inhibitor of the alternative complement pathway, which is involved in the underlying pathophysiology of IgA nephropathy<sup>4</sup>*
- *Iptacopan is in parallel development for a number of renal conditions, including IgAN, C3 glomerulopathy (C3G), atypical hemolytic uremic syndrome (aHUS), and membranous nephropathy (iMN) as well as in paroxysmal nocturnal hemoglobinuria (PNH), a hematological disease.*

**Basel, October 23, 2020** — Novartis today announced that the European Medicines Agency (EMA) has granted an orphan drug designation for iptacopan (LNP023) in IgA nephropathy (IgAN), following a recommendation from the Committee for Orphan Medicinal Products (COMP).

Orphan drug designation is granted to medicines that treat, prevent or diagnose a life-threatening or chronically debilitating rare disease, with a prevalence in the EU of below 5 in 10,000, and with either no currently approved method of diagnosis, prevention or treatment or with significant benefit to those affected by the disease.

IgAN is the most common form of primary glomerulonephritis – an inflammatory kidney disease where abnormal IgA antibody is formed, which results in immune complex deposition in the glomerular mesangium, leading to deteriorating kidney function.<sup>1,2</sup>

In patients with IgAN, proteinuria is recognized as an independent risk factor of poor prognosis. Around 30% of patients with persistent proteinuria progress to end stage renal disease (ESRD) within 10 years<sup>5</sup>. No approved targeted therapy is available.

ESRD requires dialysis or kidney transplant and is associated with significant risk of complications, considerable impact on quality of life as well as an increased risk of premature death.<sup>5</sup>

### **About iptacopan**

Iptacopan (LNP023) is a first-in-class oral, small-molecule, selective inhibitor of factor B, a key serine protease of the alternative pathway of the complement cascade mediating inflammatory responses.<sup>6-8</sup>

In addition to IgAN, iptacopan is in parallel development for a number of other renal conditions with complement system involvement where significant unmet needs exist, including C3 glomerulopathy (C3G), atypical hemolytic uremic syndrome (aHUS), and membranous nephropathy (iMN). Novartis is also investigating iptacopan in paroxysmal nocturnal hemoglobinuria (PNH), a hematological disease.

Positive Phase II data in PNH were presented at the European Society for Blood and Marrow Transplantation (EBMT) congress in August<sup>9</sup>, and Phase II interim analysis results in C3G will be presented at the upcoming virtual 2020 Annual Meeting of the American Society of Nephrology (ASN). Novartis is planning to initiate Phase III studies in several indications.

Iptacopan has the potential to become the first alternative complement pathway inhibitor to slow disease progression in a number of complement-driven diseases. Based on disease prevalence and the interim data from Phase II studies, iptacopan has also received orphan drug designations from the FDA and EMA in C3G and PNH<sup>10</sup> as well as EMA PRIME designation for C3G.<sup>11</sup>

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### **About Novartis**

Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach nearly 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 109,000 people of more than 140 nationalities work at Novartis around the world. Find out more at <https://www.novartis.com>.

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