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INVESTOR & MEDIA UPDATE

Novartis received European Medicines Agency (EMA) PRIME designation for iptacopan (LNP) in C3 glomerulopathy (C3G)

- *The European Medicines Agency has granted iptacopan a priority medicines (PRIME) designation in C3 glomerulopathy (C3G).*
- *PRIME is granted for medicines that may offer major therapeutic advance or benefit patients without treatment options.*
- *C3 glomerulopathy (C3G) is a rare renal disease, affecting young patients with a poor prognosis and significant unmet need.*[1–3]
- *Iptacopan (LNP023) is a potential first-in-class, oral, potent and selective factor B inhibitor of the complement system's alternative pathway, targeting the underlying cause of C3G.* [4–6]

Basel, October 9, 2020 — Novartis today announced that the European Medicines Agency (EMA) has granted PRIME designation for iptacopan (LNP023) in C3 glomerulopathy (C3G). PRIME is a program launched by the EMA to enhance support for the development of medicines that target an unmet medical need. This voluntary program is based on enhanced interaction and early dialogue with developers of promising medicines, to optimize development plans and speed up evaluation so these medicines can potentially reach patients earlier.

C3G is an ultra-rare and severe form of primary glomerulonephritis, characterized by complement dysregulation.[1,7] It has a worldwide annual incidence of 1–2 per million[8] and an approximate prevalence of 10,000 in the US, ~10,500 in Europe, 3,200 in Japan and 32,000 in China[9].

C3G is commonly diagnosed in adolescents and young adults. The disease has a poor prognosis; about 50% of patient progress to end-stage renal disease (ESRD) within 10 years, and 50–70% experience disease recurrence post kidney transplant.[2]

Results from a Phase II interim analysis for iptacopan in C3G will be presented at the virtually held American Society of Nephrology (ASN) 2020 Annual Meeting from October 22-25, 2020.

About iptacopan
Iptacopan is a first-in-class oral, small-molecule, reversible inhibitor of factor B, a key serine protease of the alternative pathway of the complement cascade.[4,5]

In addition to C3G, iptacopan is in parallel development for a number of other renal conditions with complement system involvement where significant unmet needs exist, including IgA nephropathy (IgAN), atypical hemolytic uremic syndrome and membranous nephropathy.

Novartis is also investigating iptacopan in paroxysmal nocturnal hemoglobinuria (PNH). Following positive Phase II data presented at the European Society for Blood and Marrow Transplantation (EBMT) congress in August[10], a randomized, active-comparator controlled open-label Phase III trial to evaluate the efficacy and safety of iptacopan in PNH patients with residual anemia despite treatment with anti-C5 antibody therapy is planned to start in Dec 2020.[11]

Iptacopan has the potential to become the first alternative pathway inhibitor to slow disease progression in a number of complement driven diseases. Based on the preliminary data from the ph2 trial, Iptacopan has received orphan designations by FDA and EMA in C3G.

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This investor update contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as “potential,” “can,” “will,” “plan,” “may,” “could,” “would,” “expect,” “anticipate,” “seek,” “look forward,” “believe,” “committed,” “investigational,” “pipeline,” “launch,” or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for the investigational or approved products described in this investor update, or regarding potential future revenues from such products. You should not place undue reliance on these statements. Such forward-looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that the investigational or approved products described in this investor update will be submitted or approved for sale or for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that such products will be commercially successful in the future. In particular, our expectations regarding such products could be affected by, among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; our ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; general political, economic and business conditions, including the effects of and efforts to mitigate pandemic diseases such as COVID-19; safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, and other risks and factors referred to in Novartis AG’s current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this investor update as of this date and does not undertake any obligation to update any forward-looking statements contained in this investor update as a result of new information, future events or otherwise.

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

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11. Novartis Data on File

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