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IR & MEDIA UPDATE

Novartis ligelizumab (QGE031) receives FDA Breakthrough Therapy designation for patients with chronic spontaneous urticaria (CSU)

- *Ligelizumab is the first treatment to receive FDA Breakthrough Therapy designation in chronic spontaneous urticaria (CSU) in patients with an inadequate response to H1-antihistamines*[1]
- *Currently there are limited approved therapies for patients with CSU, also known as chronic idiopathic urticaria (CIU)*
- *Breakthrough Therapy designation suggests ligelizumab has the potential to provide a substantial benefit over existing available treatments*
- *U.S. regulatory filing in CSU is anticipated in 2022*

Basel, January 14, 2021 — Novartis today announced that the U.S. Food and Drug Administration (FDA) has granted ligelizumab (QGE031) Breakthrough Therapy designation for the treatment of chronic spontaneous urticaria (CSU), also known as chronic idiopathic urticaria (CIU), in patients who have an inadequate response to H1-antihistamine treatment.

CSU is an unpredictable and severe disease of the skin, affecting 0.5-1% of the global population at any time[2]. It is characterized by the development of itchy, painful wheals (hives), swelling (angioedema), or both, lasting for at least 6 weeks and occurring with no known cause[3]. CSU can be challenging or frustrating for patients due to the severity and unpredictable nature[4]. It most commonly persists for 1-5 years, but in some cases even longer[2].

“Chronic spontaneous urticaria is a debilitating disease that may significantly impact a patient’s life. With so few treatment options available, patients are looking for more and better therapies to control their disease,” said Angelika Jahreis MD, PhD, Novartis Global Head Development Unit Immunology, Hepatology & Dermatology. “The FDA Breakthrough Therapy designation recognizes the need for a more effective treatment for this unpredictable, systemic and debilitating disease.”

According to FDA guidelines, treatments that receive Breakthrough Therapy Designation must target a serious or life-threatening disease and demonstrate a potential substantial improvement over existing therapies on one or more significant clinical endpoints[5].

About ligelizumab
Ligelizumab (QGE031) is a next generation monoclonal anti-immunoglobulin E (IgE) antibody. Ligelizumab is thought to work by blocking the IgE/FcεRI pathway, a key driver of the inflammatory process in CSU[6,7]. In a Phase IIb dose-finding trial, more patients experienced complete resolution of wheals (hives) with ligelizumab compared with Xolair® (omalizumab)[8]. No safety concerns were found with ligelizumab compared with omalizumab or placebo in a Phase IIb dose-finding trial in CSU patients with inadequate control on antihistamines[8]. Ligelizumab compared with omalizumab is currently being investigated in ongoing Phase III clinical trial programs including PEARL 1 and PEARL 2 (NCT03580369 and NCT03580356). The clinical trials have recruited more than 2,000 patients globally across 48 countries and results are expected in the second half of 2021[9,10].

About Novartis in CSU
Novartis is curious about the science beneath the skin and dedicated to reimagining the care of patients with diseases that can severely limit quality of life such as CSU, psoriasis, acne and atopic dermatitis. Advancing ligelizumab further strengthens the immuno-dermatology pipeline of Novartis. In the US, Novartis and Genentech, a member of the Roche Group, work together to develop and co-promote Xolair. Outside the US, Novartis markets Xolair and records all sales and related costs. Xolair is indicated as an add-on therapy for the treatment of CSU. Novartis is also testing remibrutinib (LOU064), a Bruton's tyrosine kinase (BTK) inhibitor that is being tested in Phase II clinical studies for CSU[11].

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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 110,000 people of more than 140 nationalities work at Novartis around the world. Find out more at https://www.novartis.com.

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