Novartis ligelizumab (QGE031) receives FDA Breakthrough Therapy designation for patients with chronic idiopathic urticaria (CIU)

Jan 15, 2021

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

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

CIU 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]. CIU 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 idiopathic urticaria can be a frustrating and debilitating condition for patients because the symptoms can disrupt daily life,” said Victor Bultó, President, Novartis Pharmaceuticals Corporation. “The granting of Breakthrough Therapy designation is welcome news for those who suffer from the disease because there are not many treatment options available to them. We look forward to working closely with the FDA to bring a potential new treatment option to those that need it most.”

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 (QGE031)

Ligelizumab 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 CIU[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[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)[9,10]. 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 CIU

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 CIU, 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 CIU. Novartis is also testing remibrutinib (LOU064), a Bruton's tyrosine kinase (BTK) inhibitor that is being tested in Phase II clinical studies for CIU[11].

About Novartis

Located in East Hanover, NJ Novartis Pharmaceuticals Corporation – an affiliate of 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 employs more than 15,000 people in the United States. For more information, please visit https://www.novartis.us.

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