Novartis advances ligelizumab (QGE031) in urticaria to Phase III on basis of strong Phase II head-to-head data

- **PEARL 1 and PEARL 2**, the largest pivotal trials to date in chronic spontaneous urticaria (CSU), will enroll more than 2,000 CSU patients\(^1,2\)

- Ligelizumab (QGE031), a monoclonal antibody, is being developed as a treatment option for CSU patients whose symptoms are inadequately controlled by H1-antihistamines\(^3\)

- In a head-to-head study, ligelizumab showed improvements over Xolair® (omalizumab) in CSU patients\(^4\)

**Basel, December 4, 2018** – Novartis, a leader in immuno-dermatology, announced today the initiation of Phase III trials for ligelizumab (QGE031) – a high-affinity monoclonal anti-IgE antibody – in chronic spontaneous urticaria (CSU) patients whose symptoms are inadequately controlled by H1-antihistamines\(^1,2\). Phase III studies PEARL1 and PEARL 2 are planned to include more than 2,000 CSU patients\(^1,2\).

“CSU has a big impact on patients’ lives,” said Marcus Maurer, MD, Professor of Dermatology and Allergy and Director of Research at the Department of Dermatology and Allergy, Allergie-Centrum-Charité of the Charité-Universitätsmedizin in Berlin, Germany. “Despite existing treatment options, too many people continue to struggle with the debilitating and potentially painful symptoms of CSU. Advancing ligelizumab to Phase III is encouraging news for physicians and patients who have difficulty in controlling symptoms.”

Results from the placebo- and active-controlled Phase IIb trial showed that ligelizumab met the primary endpoint by demonstrating a clear dose-response relationship, and improvements over Xolair® (omalizumab) in CSU patients\(^4\). Ligelizumab achieved rapid onset of action and improved and sustained efficacy in CSU patients, whose symptoms are not adequately controlled by H1-antihistamines\(^4\).

“Novartis is committed to leveraging our strong heritage and expertise in immuno-dermatology to reimagine and discover potential new treatments which can benefit patients,” said Eric Hughes, Global Development Unit Head, Immunology, Hepatology and Dermatology. “By initiating ligelizumab to Phase III studies we continue to honor that commitment.”

The purpose of Phase III studies PEARL 1 and PEARL 2 is to establish efficacy and safety of ligelizumab in adolescent and adult patients ≥ 12 years of age with CSU who remain symptomatic despite the use of H1-antihistamines\(^1,2\). Both trials are multi-center, randomized, double-blind, active- and placebo-controlled, parallel-group studies\(^1,2\) in 48 countries including the US, Germany and Japan\(^5\).

**About Novartis in CSU**
Advancing ligelizumab further strengthens the immuno-dermatology pipeline of Novartis. Novartis currently also markets Xolair. Xolair, indicated as an add-on therapy for the treatment
of CSU, is the only therapy recommended by the global guideline on chronic urticaria (CU) for patients unresponsive to antihistamines. In the US, Novartis Pharmaceuticals Corporation and Genentech work together to develop and co-promote Xolair.

**About CSU**

CSU is a severe, unpredictable skin condition that, if not or only partially controlled, has a major impact on the quality of sleep and the social and working lives of patients. Symptoms include spontaneous swelling of the skin and the appearance of itchy hives which can have a profoundly negative impact on patients’ sleep, work productivity and subsequent health-related quality of life. Recent data publications suggested that some patients with CSU still report high disease burden despite previous treatment with urticaria medication such as H1-/H2-antihistamines or montelukast, with two thirds still having severe activity (UAS7 ≥28) and more than half reporting a very large effect of CSU on their life (DLQI >10). Employed CSU patients also report a high negative impact on their work.

**About PEARL 1 and PEARL 2**

PEARL 1 and 2 are Phase III, multi-center, randomized, double-blind, active- and placebo-controlled, parallel-group studies designed to establish efficacy and safety of ligelizumab in adolescent and adult subjects with CSU who remain symptomatic despite H1-antihistamine treatment by demonstrating better efficacy over Xolair (omalizumab). More than 2,000 patients will initially be randomized to ligelizumab dose A, ligelizumab dose B, omalizumab 300mg with treatment given every 4 weeks for one year. Patients initially randomized to placebo will be switched to ligelizumab dose B starting week 24 until week 52. The primary outcome will measure absolute change from baseline in UAS7 at Week 12.

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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 1 billion people globally and we are finding innovative ways to expand access to our latest treatments. About 125 000 people of more than 140 nationalities work at Novartis around the world. Find out more at www.novartis.com.

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References
5. Data on file

Friedrich von Heyl
Novartis Global Pharma Communications
+41 61 324 8984 (direct)
+41 79 749 0286 (mobile)
friedrich.vonheyl@novartis.com