Benlysta granted Orphan Drug Designation by US FDA for the potential treatment of systemic sclerosis

PUBLISHED JAN 31, 2023
BY GSK

For media and investors only

GSK plc (LSE/NYSE: GSK) today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to Benlysta (belimumab), a B-cell inhibiting monoclonal antibody, for the potential treatment of systemic sclerosis. GSK plans to initiate a phase II/III trial of belimumab for systemic sclerosis associated interstitial lung disease (SSc-ILD) in the first half of 2023.

Systemic sclerosis (SSc) is a rare autoimmune disease that causes atypical growth of connective tissues and can affect the musculoskeletal system, heart, lungs, kidneys, skin, and other organs. Interstitial lung disease (ILD) is the leading cause of death in SSc, affecting as many as half of people living with the disease. 1, 2

With limited treatment options available for SSc-ILD, this Orphan Drug Designation reflects the need for further research and the potential for belimumab to address a critical need for people living with this debilitating condition. GSK continues to follow the science to explore how belimumab may be able to address an unmet need in B-cell-driven autoimmune diseases.

The US FDA's ODD is a special status granted to support the development and evaluation of potential new medicines intended for the treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US.

About Benlysta (belimumab)

Benlysta (belimumab) is a B-lymphocyte stimulator (BLyS) specific inhibitor that binds to soluble BLyS, which is found to be increased in

patients with systemic autoimmune diseases like systemic lupus erythematosus (SLE) and lupus nephritis (LN). 3 A fully human monoclonal antibody, Benlysta inhibits the prolonged survival of B cells induced by increased BLyS, including autoreactive B cells, and reduces the differentiation of B cells into immunoglobulin-producing plasma cells. The US FDA first approved Benlysta for the treatment of active SLE; it is the first and only approved biologic for both SLE and LN in more than 50 years, including for the paediatric population.

Please see the US Prescribing Information for BENLYSTA(PDF - 3,702KB).

Research indicates that elevated BLyS and autoreactive B cells play a central role in the pathogenesis of SSc, a rare autoimmune disease affecting 2.3-10 people per million. 4, 5 SSc is characterised by microvascular damage, dysregulation of immunity and progressive fibrosis in multiple organs. 2, 6 ILD is a common and serious complication, marked by inflammation and scar tissue build-up in the lungs. ILD is observed in as many as half of SSc patients and is a significant contributor to patients' disease burden and mortality. 1 There is a recognised need for additional effective, well-tolerated, disease-modifying treatment options for SSc-ILD. 2

GSK is a global biopharma company with a purpose to unite science, technology, and talent to get ahead of disease together. Find out more at gsk.com/company.

Cautionary statement regarding forward-looking statements

GSK cautions investors that any forward-looking statements or projections made by GSK, including those made in this announcement, are subject to risks and uncertainties that may cause actual results to differ materially from those projected. Such factors include, but are not limited to, those described in the Company's Annual Report on Form 20-F for 2021, GSK's Q4 Results for 2022 and any impacts of the COVID-19 pandemic.

1 Vonk MC, Smith V, Sfikakis PP, Cutolo M, Del Galdo F, Seibold JR. Pharmacological treatments for SSc-ILD: Systematic review and critical appraisal of the evidence. Autoimmun Rev. 2021 Dec;20(12):102978. doi: 10.1016/j.autrev.2021.102978. Epub 2021 Oct 28. PMID: 34718159. Available at: https://www.sciencedirect.com/science/article/pii/S15689972210.... 2

Fischer A, Patel NM, Volkmann ER. Interstitial Lung Disease in Systemic Sclerosis: Focus on Early Detection and Intervention. Open Access Rheumatol. 2019 Dec 9;11:283-307. doi: 10.2147/OARRR.S226695. PMID: 31849543; PMCID: PMC6910104. Available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6910104/. 3 Parodis I, Zickert A, Sundelin B, et alEvaluation of B lymphocyte stimulator and a proliferation inducing ligand as candidate biomarkers in lupus nephritis based on clinical and histopathological outcome following induction therapy Lupus Science & Medicine 2015;2:e000061. doi: 10.1136/lupus-2014-000061. 4 Thoreau, B., Chaigne, B., & Mouthon, L. (2022). Role of B-Cell in the Pathogenesis of Systemic Sclerosis. Frontiers in immunology, 13, 933468. https://doi.org/10.3389/fimmu.2022.933468. 5 Ghosh, S. K., Bandyopadhyay, D., Saha, I., & Barua, J. K. (2012). Mucocutaneous and demographic features of systemic sclerosis: a profile of 46 patients from eastern India. Indian journal of dermatology, 57(3), 201–205. https://doi.org/10.4103/0019-5154.96193. 6 Truchetet ME, Brembilla NC, Chizzolini C. Current Concepts on the Pathogenesis of Systemic Sclerosis. Clin Rev Allergy Immunol. 2021 Sep 6. doi: 10.1007/s12016-021-08889-8. Epub ahead of print. PMID: 34487318. Available at: https://link.springer.com/article/10.1007/s12016-021-08889-8.

Press release distributed by Wire Association on behalf of GSK, on Jan 31, 2023. For more information subscribe and follow us.

Media Assets

Embedded Media

Visit the online press release to interact with the embedded media.

https://wireassociation.eu/newsroom/gsk/releases/en/benlysta-granted-orphan-drug-designation-by-us-fda-for-the-potential-treatment-of-systemic-sclerosis-1785

GSK

Newsroom: https://wireassociation.eu/newsroom/gsk
Website: https://www.gsk.com/
Primary Email: corporate.media@gsk.com

Social Media

Facebook - https://www.facebook.com/GSK
Twitter - http://twitter.com/GSK
Youtube - http://www.youtube.com/GSK
Linkedin - http://www.linkedin.com/company/glaxosmithkline
Instagram - https://www.instagram.com/gsk/