U.S. FDA Grants Orphan Drug Designation to Gamgertamig for the Treatment of Pemphigus

Mechelen, Belgium; 24 August 2026, 22.01 CET; regulated information – Lakefront Biotherapeutics NV (Euronext & NASDAQ: LKFT) today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to gamgertamig for the treatment of pemphigus. Gamgertamig was previously granted both Fast Track and Orphan Drug Designation by the U.S. FDA for the treatment of autoimmune hemolytic anemia (AIHA) and immune thrombocytopenia (ITP).

Gamgertamig is being developed by Lakefront in collaboration with Gilead Sciences. Ongoing clinical trials in autoimmune diseases are actively enrolling, and expansion of the clinical trials program is anticipated in 2027.

- Phase 1b study with gamgertamig in patients with autoimmune cytopenias (including ITP and AIHA) (NCT07083960), active in Australia and the United States.
- Phase 1b study with gamgertamig in seropositive autoimmune diseases (including idiopathic inflammatory myositis, Sjögren's disease, pemphigus vulgaris (PV), and pemphigus foliaceus (PF)) (NCT07229144) active in Australia, New Zealand, Czech Republic, and Japan.
- Expect to initiate proof-of-concept basket studies assessing gamgertamig in additional autoimmune indications.

"We're excited by the U.S. FDA's award of Orphan Drug Designation for gamgertamig for the treatment of pemphigus as we continue to advance it for patients suffering with this debilitating and potentially life-threatening autoantibody-driven disease," said Eric Hedrick MD, Chief Medical Officer of Lakefront. "I continue to believe gamgertamig has the potential to represent a transformative medicine in autoimmune diseases."

About Gamgertamig

Gamgertamig is an investigational BCMAxCD3 bispecific T cell engager for the treatment of autoantibodies driven immune-mediated disease. Gamgertamig has been granted Orphan Drug Designation and Fast Track Designation by the U.S. FDA for certain autoimmune diseases. Gamgertamig is currently in Phase 2 studies and is expected to enter registrational studies in 2027. Gamgertamig is in-licensed from Keymed Biosciences, which owns the rights to develop the program in Greater China.

About Lakefront Biotherapeutics

Lakefront Biotherapeutics is a biotechnology company dedicated to building a differentiated pipeline of medicines for patients with serious diseases in areas of high unmet need. The Company has established a clinical-stage portfolio in immunology and inflammation, anchored by gamgertamig, a potential first-in-class and best-in-class BCMAxCD3 T-cell engager for autoimmune diseases. Backed by deep deal-making expertise, operational flexibility, and a strong capital position, Lakefront identifies, acquires, and advances high-quality assets with clear potential to deliver meaningful patient impact and long-term shareholder value. For more information, visit https://www.lakefrontbio.com or follow us on LinkedIn or X.

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Forward-looking statements

This press release includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. These statements are often, but are not always, made through the use of words or phrases such as "anticipate," "expect," "plan," "estimate," "will," "continue," "aim," "intend," "future," "potential," "could," "indicate," "forward," as well as similar expressions. Forward-looking statements contained in this release include, but are not limited to, statements regarding gamgertamig's phase 1b and proof-of-concept basket studies, statements regarding the expected timing, design and readouts of the gamgertamig studies, and statements regarding the potential benefits of gamgertamig. Forward-looking statements involve known and unknown risks, uncertainties and other factors which might cause Lakefront's actual results to be materially different from those expressed or implied by such forward-looking statements. These risks, uncertainties and other factors include, without limitation, the risk that preliminary or interim clinical results may not be replicated in ongoing or subsequent clinical trials, the risk that ongoing and future clinical studies with gamgertamig may not be completed in the currently envisaged timelines or at all, the inherent uncertainties associated with competitive developments, clinical trial and product development activities and regulatory approval requirements (including that data from the ongoing and planned clinical development program may not support registration or further development of gamgertamig due to safety, efficacy, or other reasons), Lakefront's reliance on collaborations with third parties, and that Lakefront's estimations regarding its gamgertamig clinical program and regarding the commercial potential of gamgertamig may be incorrect, as well as those risks and uncertainties identified in Lakefront's Annual Report on Form 20-F for the year ended December 31, 2025 filed with the U.S. Securities and Exchange Commission (SEC) and its subsequent filings with the SEC. All statements other than statements of historical fact are statements that could be deemed forward-looking statements. The forward-looking statements contained herein are based on management's current expectations and beliefs and speak only as of the date hereof, and Lakefront makes no commitment to update or publicly release any revisions to forward-looking statements in order to reflect new information or subsequent events, circumstances, or changes in expectations, unless specifically required by law or regulation.