TG Therapeutics Receives Orphan Drug Designation for Umbralisib from the U.S. Food and Drug Administration for the Treatment of Marginal Zone Lymphoma

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NEW YORK, April 15, 2019 (GLOBE NEWSWIRE) -- TG Therapeutics, Inc. (NASDAQ: TGTX) today announced that the U.S. Food and Drug Administration (FDA) granted orphan drug designation to its phosphoinositide-3-kinase (PI3K) delta inhibitor, umbralisib (TGR-1202), for the treatment of patients with all three types of marginal zone lymphoma (MZL): nodal, extranodal, and splenic MZL.

Umbralisib monotherapy is being evaluated in the UNITY-NHL Phase 2b registration directed clinical trial. The MZL cohort of the UNITY-NHL trial is currently evaluating the safety and efficacy of single agent umbralisib in patients with MZL who have received at least one prior anti-CD20 regimen, the same indication for which the FDA recently granted breakthrough therapy designation for umbralisib.

Michael S. Weiss, Executive Chairman and Chief Executive Officer of TG Therapeutics, stated “Receiving orphan drug designation for umbralisib in patients with MZL is another important milestone in our commitment to developing novel treatment options for patients with B-cell malignancies, including orphan diseases such as MZL.” Mr. Weiss continued, “We are highly encouraged by the interim results presented thus far for the MZL cohort of the UNITY-NHL trial and we look forward to presenting final data from this cohort later this year and to discussing the results with the FDA with the goal of filing for accelerated approval of umbralisib by year-end.”

ABOUT ORPHAN DRUG DESIGNATION

Orphan drug designation is granted by the U.S. FDA to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S. Orphan drug designation provides certain incentives which may include tax credits towards the cost of clinical trials and prescription drug user fee waivers. If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity.

ABOUT MARGINAL ZONE LYMPHOMA

Marginal zone lymphoma (MZL) comprises a group of indolent (slow growing) B-cell non-Hodgkin lymphomas (NHLs) that begin forming in the marginal zone of lymphoid tissue. With an annual incidence of approximately 7,500 newly diagnosed patients, MZL is the third most common B-cell NHL accounting for approximately eight percent of all NHL cases. MZL consists of three different subtypes: extranodal MZL, nodal marginal zone lymphoma (NMZL), and splenic marginal zone lymphoma (SMZL).

ABOUT THE UNITY-NHL PHASE 2b STUDY—Marginal Zone Lymphoma Cohort

The multicenter, open-label, UNITY-NHL Phase 2b study - Marginal Zone Lymphoma cohort was designed to evaluate the safety and efficacy of single agent umbralisib in patients with MZL who have received at least one prior anti-CD20 regimen. The primary endpoint is overall response rate (ORR) as determined by Independent Review Committee (IRC) assessment. Secondary endpoints include safety, duration of response, and progression-free survival (PFS).

The MZL cohort completed enrollment in August 2018, and in February of 2019 the Company announced that the primary endpoint of ORR as determined by central IRC was met for all treated patients (n=69).

Earlier this month at the American Association of Cancer Research (AACR) annual meeting, interim data from the UNITY-NHL MZL cohort were presented on the first 42 patients enrolled, demonstrating an ORR of 52%, with a Complete Response (CR) rate of 19%, and a tolerable safety profile amongst all patients treated to date (n=69).

ABOUT TG THERAPEUTICS, INC.

TG Therapeutics is a biopharmaceutical company focused on the acquisition, development and commercialization of novel treatments for B-cell malignancies and autoimmune diseases. Currently, the company is developing multiple therapies targeting hematological malignancies and autoimmune diseases. Ublituximab (TG-1101) is a novel, glycoengineered monoclonal antibody that targets a specific and unique epitope on the CD20 antigen found on mature B-lymphocytes. TG Therapeutics is also developing umbralisib (TGR-1202), an oral, once-daily inhibitor of PI3K-delta. Umbralisib uniquely inhibits CK1-epsilon, which may allow it to overcome certain tolerability issues associated with first generation PI3K-delta inhibitors. Both ublituximab and umbralisib, or the combination of which is referred to as "U2", are in Phase 3 clinical development for patients with hematologic malignancies, with ublituximab also in Phase 3 clinical development for Multiple Sclerosis. Additionally, the Company has recently brought into Phase 1 clinical development, TG-1501, its anti-PD-L1 monoclonal antibody, TG-1701, its covalently-bound Bruton’s Tyrosine Kinase (BTK) inhibitor and TG-1801, its anti-CD47/CD19 bispecific antibody. TG Therapeutics is headquartered in New York City.

Cautionary Statement
Some of the statements included in this press release may be forward-looking statements that involve a number of risks and uncertainties. For those statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995. In addition to the risk factors identified from time to time in our reports filed with the Securities and Exchange Commission, factors that could cause our actual results to differ materially are the following: the risk that the interim data (the “Interim Results”) from the UNITY-NHL MZL cohort presented during AACR will not be reproduced when the final analysis is conducted on all patients later this year, including the risk that the final results will demonstrate a lower ORR and/or enhanced toxicities, which may not support a filing for accelerated approval; the risk that even if the Interim Results

are reproduced in the final analysis of the UNITY-NHL MZL cohort or that the final results otherwise meet the Company's target ORR of 40-50%, that the final results will still be insufficient to support a filing for accelerated approval; the risk that umbralisib will not receive accelerated approval based on data from the UNITY-NHL MZL cohort even if the final results are deemed positive by the Company and support a filing for accelerated approval; the risk that the positive Interim Results from the UNITY-NHL MZL cohort will not be reproduced in other cohorts of the UNITY-NHL study or in other studies being conducted by the Company; the risk that duration of response or progression free survival data from the UNITY-NHL cohort when available for all patients will not be positive or supportive of accelerated approval; the risk that safety issues will arise when the final safety data are cleaned and analyzed for all patients in the UNITY-NHL MZL cohort; the risk that our belief that umbralisib has a differentiated safety profile will not be shared by physicians or the FDA or will not be reproduced in the final analysis of the UNITY-NHL MZL cohort, in other cohorts of the UNITY-NHL study, in the UNITY-CLL study or in any other of our on-going studies; the risk that we are not able to successfully and cost effectively complete all the preclinical, clinical and CMC requirements necessary to support accelerated approval. Any forward-looking statements set forth in this press release speak only as of the date of this press release. We do not undertake to update any of these forward-looking statements to reflect events or circumstances that occur after the date hereof. This press release and prior releases are available at www.tgtherapeutics.com. The information found on our website is not incorporated by reference into this press release and is included for reference purposes only.

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