Oncotelic Initiates Clinical Trials Evaluating OT-101 against Pediatric Gliomas

AGOURA HILLS, Calif., Nov. 08, 2022 (GLOBE NEWSWIRE) -- Oncotelic Therapeutics, Inc (OTCQB:OTLC) ("Oncotelic", the "Company" or "We"), a developer of treatments for rare and orphan indications, including Parkinson's Disease, PDAC, DIPG, and COVID-19, today announced that it has submitted a clinical study protocol to the US Food and Drug Administration ("FDA") for the initiation of a Phase 1 Trial (designated "G101") for OT-101, the Company's transforming growth factor beta 2 ("TGF-β2") inhibitor, as a treatment for patients with recurrent/relapsed DMG.

G101: An Open-label Dose Escalation Study to Evaluate the Safety and Tolerability of Repeated Cycles of OT-101 in Pediatric Diffuse Midline Glioma ("DMG") Patients, Administered Intraventricularly.

OT-101 is a first-in-class anti-TGF-β2 ribonucleic acid ("RNA") therapeutic that has exhibited single agent activity in relapsed/refractory cancer patients in multiple clinical trials. OT-101 has also demonstrated activity against the COVID-19 virus in our Phase 2 clinical trial- C001.

"Pediatric DMG is a very aggressive brain tumor in children that has a dismal prognosis with a median overall survival of less than one year after standard radiation therapy. Therefore, there is an urgent need for therapeutic innovations for treatment of pediatric DMG", explained Fatih Uckun, MD PhD, the Chief Medical Officer of Oncotelic. "The primary goal of the new study is to carefully evaluate the safety and tolerability of OT-101 when it is administered directly into the cerebral spinal fluid (CSF) of pediatric patients with DMG", he added.

"This is the first of a series of planned clinical trials in pediatric patients with gliomas evaluating clinical benefit while also assessing predictive biomarkers." Dr. Vuong Trieu, CEO and Chairman of Oncotelic. "The groundwork laid down by our successful clinical program in adult gliomas and deep datamining by the team will now help guide the development of OT-101 for pediatric DMG."

About DMG/DIPG

DMG is a highly morbid pediatric central nervous system (CNS) tumor for which there is currently no effective treatment. DMG is responsible for 50% of all childhood high grade glioma ("HGG"). Due to their anatomic location and infiltrative nature, DMGs are not amenable to surgical resection and are most often diagnosed radiographically and treated with radiation therapy, with no effect on survival.

Specifically, 80% of pediatric DMGs harbor somatic mutations in histone H3-encoding genes H3F3A (60%), HIST2H3C or HIST1H3B/C (20%), resulting in lysine-27-to-methionine (H3K27M) conversion that confers a more aggressive clinical course and poorer overall response to therapy. As such, DMG, H3 K27-mutant was first introduced in the 2016 World Health Organization ("WHO") classification of CNS tumors as a newly defined entity and these tumors are WHO grade 4 gliomas associated with local infiltration and a poor prognosis with 2-year survival rate of < 10%, regardless of histological grading. This entity represents the majority of DIPGs as well as tumors found along the midline (e.g., brainstem, midbrain, thalamus, and spine).

About OT-101

OT-101, is a first-in-class anti-TGF-β2 RNA therapeutic that exhibited single agent activity in some relapsed/refractory cancer patients in clinical trial settings. HGGs are characterized by a T-cell exhaustion signature and pronounced T-cell hypo responsiveness of their tumor microenvironment ("TME"). TGF-β2 has been implicated as a key contributor to the immunosuppressive landscape of the TME in HGG. OT-101 is designed to abrogate the immunosuppressive actions of TGF- β2. In a completed Phase 2 clinical study, OT-101 exhibited clinically meaningful single-agent activity and induced durable complete and partial responses in recurrent and refractory adult HGG patients, including young adults with Glioblastoma Multiforme or Amyloidosis.

OT-101 has been granted orphan designation by the FDA under the Orphan Drug Act ("ODA"). ODA provides for granting special status to a drug to treat a rare disease or condition upon request of a drug company. Orphan designation qualifies the sponsor of the drug for various development incentives of the ODA, including tax credits for qualified clinical testing. OT-101 also been granted Rare Pediatric Designation for DIPG. The FDA grants rare pediatric disease designation for diseases with serious or life-threatening manifestations that primarily affect people aged from birth to 18 years, and that affect fewer than 200,000 people in the U.S. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application or biologics license application for a product for the prevention or treatment of a rare pediatric disease may be eligible for a voucher, which can be redeemed to obtain priority review for any subsequent marketing application and may be sold or transferred.

As previously reported, on March 31, 2022, we entered into a joint venture, or JV, with Dragon Overseas Capital Ltd. (Dragon Overseas) and GMP Biotechnology Ltd. (GMP Bio). The JV and Oncotelic will develop and ultimately market OT-101, individually and/or in combination with other products. Oncotelic would receive up to $50 million on sale of the RPD voucher, following marketing approval of OT-101 for diffuse intrinsic pontine glioma, or DIPG, by the US Food and Drug Administration.

Oncotelic's Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Al statements, other than statements of historical facts, included in this communication regarding strategy, future operations, future financial position, prospects, plans and objectives of management are forward-looking statements. Words such as "may", "expect", "anticipate" "hope", "vision", "optimism", "design", "exciting", "promising", "wil", "conviction", "estimate," "intend," "believe", "quest for a cure of cancer", "innovation-driven", "paradigm-shift", "high scientific merit", "impact potential" and similar expressions are intended to identify forward-looking statements. Forward looking statements contained in this press release include, but are not limited to, statements about future plans related to the development of our product pipeline for animal health products, use of blockchain technology to enhance the drug development process, scope and success of our product development efforts in the animal health space and the potential use of the company's product candidates to treat various cancer indications as well as obtaining required regulatory approval to conduct clinical trials and upon granting of approval by the regulatory agencies, the successful marketing of the products. Each of these forward-looking statements involves risks and uncertainties, and actual results may differ materially from these forward-looking statements or may not occur at a l. Many factors may cause differences between current expectations and actual results, including unexpected safety or efficacy data observed during preclinical or clinical studies, clinical trial site activation or enrolment rates that are lower than expected, changes in expected or existing competition, changes in the regulatory environment, failure of collaborators to support or advance collaborations or product candidates and unexpected litigation or other disputes, taking the Company or its affiliates through initial public offerings. These risks are not exhaustive, the company faces known and unknown risks,

including the risk factors described in the Company's annual report on Form 10-K filed with the SEC on April 15, 2022 and in the company's other periodic filings. Forward-looking statements are based on expectations and assumptions as of the date of this press release. Except as required by law, the company does not assume any obligation to update forward-looking statements contained herein to reflect any change in expectations, whether as a result of new information future events, or otherwise.

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