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GT Biopharma Announces Completion of Dosing in Phase 1 Clinical Trial of GTP-004 for Myasthenia Gravis

LOS ANGELES, January 9, 2018 /PRNewswire/ --

GT Biopharma Inc. (OTCQB: GTBP) and (Euronext Paris: GTBP.PA) today announced that it has completed dosing in its Phase 1 clinical trial for GTP-004, its promising treatment for the symptoms of myasthenia gravis. The results provide evidence that GTP-004 enables the safe and well-tolerated administration of doses of pyridostigmine.

GTP-004 combines pyridostigmine with ondansetron, designed to attenuate the gastro-intestinal (GI) side effects of pyridostigmine alone, providing the potential for a fully efficacious dose of pyridostigmine to be safely used. Based on the data, and discussions with key opinion leaders, GT Biopharma expects to be in a position to initiate a Phase 2 clinical trial in patients in the second half of 2018.

The objective of the Phase 1 clinical trial is to demonstrate that GI side effects are safely reduced with GTP-004. Five healthy volunteers were enrolled in the Phase 1 study. Following enrollment, subjects received single increasing oral doses of pyridostigmine (ranging from 30 to 120mg) administered once daily in the morning. Once subjects reached First Intolerable Dose (FID1) as defined by protocol criteria, upward dose escalation of pyridostigmine was discontinued and subjects were washed out for 2 to 7 days. Next, subjects that reached FID received daily increasing doses of pyridostigmine in combination with ondansetron.

Three subjects (2 males, one female; aged 34 to 43) reached FID with pyridostigmine alone. The dose-limiting gastro-intestinal adverse event occurred at 60 mg for 2 subjects, and 90 mg for the third subject. When these three subjects received GTP-0004 (pyridostigmine with ondansetron), gastro-intestinal adverse events were abrogated, and all subjects tolerated doses as high as 120 mg, the maximum allowed dose allowed by the protocol.

GT Biopharma owns the worldwide rights to commercialize GTP-004 and intellectual property protection including composition of matter patents on the combination.

GT Biopharma Chief Executive Officer Dr. Kathleen Clarence-Smith said, "These are early results, but the data provide evidence of the ability of GTP-004 to avoid the GI side effects of administering pyridostigmine alone and offer hope to all those suffering from myasthenic syndromes. We expect to be in a position to begin a Phase 2 clinical trial in patients in the second half of 2018."

About Myasthenia Gravis

Myasthenia gravis is a rare autoimmune muscle disease caused by antibodies that attack certain components of muscles leading to varying degrees of weakness and fatigue. The prevalence of myasthenia gravis in the United States is estimated at 14 to 20 per 100,000 population, approximately 36,000 to 60,000 cases in the US (Howard, 2015). The hallmark of the disease is muscle weakness that increases during periods of activity and improves after periods of rest, involvement of the bulbar and respiratory muscles can be life-threatening (Phillips and Vincent, 2016). The disease occurs in all ethnic groups and both genders. Onset commonly occurs in young adult women (under 40 years) and older men (over 60 years), but it can occur at any age (NINDS, 2017).

The treatment of the symptoms of myasthenia gravis involves treatment of the muscle weakness by acetylcholinesterase inhibitors such as pyridostigmine that do not cross the blood-brain barrier (Gotterer and Li, 2016). Pyridostigmine is associated with considerable improvement in muscle strength in some patients and little to none in others (Howard, 2015). The use of pyridostmine, however, is plagued by the occurrence of intolerable gastro-intestinal side effects (vomiting, nausea, diarrhea), that limit achievable efficacy.

About GT Biopharma, Inc.

GT Biopharma, Inc. is a biotechnology company focused on innovative drugs for the treatment of cancer and nervous system diseases (Neurology and Pain) along with other unmet medical needs. GT's lead oncology drug candidate, OXS-1550 (DT2219) is a novel bispecific scFv recombinant fusion protein-drug conjugate composed of the variable regions of the heavy and light chains of anti-CD19 and anti-CD22 antibodies and a modified form of diphtheria toxin as its cytotoxic drug payload. OXS-1550 targets cancer cells expressing the CD19 receptor or the CD22 receptor or both receptors. When OXS-1550 binds to cancer cells, the cancer cells internalize the drug and are killed due to the action of cytotoxic payload. OXS-1550 has demonstrated success in early human clinical trials in patients with relapsed/refractory B-cell lymphoma or leukemia. In addition, GT's TriKE platform will address a number of cancer types. GT's nervous system platform is focused on acquiring or discovering and patenting late-stage, de-risked, and close-to-market improved treatments for nervous system diseases (Neurology and Pain) and shepherding them through the approval process to the NDA. GT Biopharma's neurology products currently include PainBrake, as well as treatments for the symptoms of myasthenia gravis, and motion sickness.

Except for historical information contained herein, the statements in this release are forward-looking and made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are inherently unreliable and actual results may differ materially. Examples of forward-looking statements in this news release include statements regarding the effectiveness of the Company's products, the potential outcome of clinical studies, the future success of development activities and the future growth and operating and financial performance of the Company. Factors which could cause actual results to differ materially from these forward-looking statements include such factors as the Company's ability to accomplish its business initiatives, obtain regulatory approval and protect its intellectual property; significant fluctuations in marketing expenses and ability to achieve or grow revenue, or recognize net income, from the sale of its products and services, as well as the introduction of competing products, or management's ability to attract and maintain qualified personnel necessary for the development and

commercialization of its planned products, and other information that may be detailed from time to time in the Company's filings with the United States Securities and Exchange Commission. The Company undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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