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Takeda acquires license for celiac disease therapy

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From COUR Pharmaceuticals Following Positive Phase 2a Proof-of-Concept Study

Japanese firm Takeda Pharmaceutical Company and US based COUR Pharmaceutical Development Company, Inc. have announced that Takeda has acquired an exclusive global license to develop and commercialize the investigational medicine CNP-101/TAK-101, an immune modifying nanoparticle containing gliadin proteins. Based on COUR's antigen specific immune tolerance platform, TAK-101 is a potential first-in-class treatment targeting the aberrant immune response in celiac disease, a serious autoimmune disease where the ingestion of gluten leads to inflammation and damage in the small intestine.

Results of a randomized, double-blind, placebo-controlled clinical trial to assess the markers of potential efficacy and safety of the investigational medicine in 34 adults with proven celiac disease was presented today as a late-breaking abstract at UEG Week 2019, Barcelona, Spain. At inclusion, patients had well-controlled biopsy proven celiac disease. After inclusion, they underwent an oral gluten challenge. Based on the study, Takeda exercised its option to acquire the exclusive global license to TAK-101.

"While many people living with celiac disease can manage their symptoms by following a gluten free diet, there are currently no treatment options for those who continue to have symptoms," said Asit Parikh M.D., Ph.D., Head, Gastroenterology Therapeutic Area Unit at Takeda. "Our collaboration with COUR has shown, for the first time, that it is possible to induce specific immune tolerance to a foreign antigen in autoimmune diseases such as celiac disease. With our expertise in inflammatory diseases, Takeda is well positioned to further develop TAK-101 in pursuit of providing the first approved treatment option for patients with celiac disease."

In the trial, treatments were administered intravenously on day 1 and day 8. The gluten challenge began seven days after the second treatment administration and included 12 grams of gluten per day for three days followed by 6 grams of gluten per day for 11 days. The primary endpoint was change from baseline in interferon-gamma (IFN-?) spot forming units (SFUs) at day 6 after gluten challenge using a gliadin-specific enzyme-linked immunospot (ELISpot) assay. This test is a direct measure of gluten-specific systemic T cell activation in celiac disease, and blocking this response suggests individuals with celiac disease could be protected from the effects of gluten exposure. 34 patients were randomized and treated, 6 discontinued due to gluten related symptoms, and 28 completed the 14-day gluten challenge per protocol.

The primary endpoint of the trial was achieved with a mean change from baseline in IFN-? ELISpotSFUs of 2.10 and 17.57 with TAK-101 and placebo, respectively (p=0.0056). Also seen was a trend in protection from small intestinal mucosal damage with deterioration of 0.18 with TAK-101 compared with 0.63 with placebo (p=0.079). The most frequent adverse events in patients receiving TAK-101 that exceeded the frequency seen in placebo treated patients were nausea, headache, abdominal pain, and back pain. No patient had clinically significant changes in vital signs, routine clinical labs, or serum cytokines/chemokines, gliadin-specific T cell proliferation and cytokine secretion.

Takeda intends to initiate a dose-ranging study to further explore the potential of TAK-101 in the treatment of patients with celiac disease on a gluten free diet to inform future registrational trials. COUR is eligible to receive up to $420 million in future payments, and royalties on sales of any commercialized products resulting from the license.

“We are encouraged by the data from this first human proof of concept study of our proprietary nanoparticle platform designed to reprogram the immune system,” said John J. Puisis, CEO of COUR Pharmaceuticals. “As Takeda assumes responsibility for the celiac disease program, COUR will focus on advancing our pipeline of therapies for a variety of other immune disorders ranging from multiple sclerosis to peanut allergy.”

COUR’s proprietary immune modifying nanoparticles bind inflammatory cells to initiate tolerogenic immune reprogramming. The interior core can be loaded with disease specific antigen – in this case, gliadin proteins – to induce tolerance in autoimmune conditions like celiac disease.