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## A phase IIb clinical study of tissue gene-C (TG-C) in patients with osteoarthritis

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 $T^{G-C}$  is a gene and cell therapy for osteoarthritis that employs ex-vivo gene delivery via a retrovirally transduced chondrocytes that overexpress transforming growth factor- $\beta$ 1 (TGF- $\beta$ 1). TG-C contains non-transformed (hChonJ) and transduced (hChonJb#7) chondrocytes by the ratio of 3:1.

The randomized single blind, placebo-controlled phase IIb trials were conducted to determine both safety and efficacy in patients with knee osteoarthritis. Participants (n=54) with a confirmed diagnosis of knee osteoarthritis by X-ray and MRI were randomized into the treatment group (TG-C, n=27) and the control group (saline, n=27). The primary parameter was evaluated by International Knee Document Committee (IKDC) which measures pain, sports activities, and daily function. The secondary parameters were evaluated by Western-Ontario and MacMaster University (WOMAC) score, Knee Injury and Osteoarthritis Outcome Score (KOOS), 100 mm Visual Analogue Scale (VAS) and MRI (dGEMRIC and T2). These parameters were evaluated at 12, 24 and 48 weeks post treatment.

Additionally, changes in biomarkers were assessed in serum and urine samples. Safety measures, including physical exams, complete blood count, and serum chemistry, were included up to 6 months post treatment. Blood samples were screened to detect the replication competent retrovirus (RCR), retrovirally transduced cells, and TGF- $\beta$ 1 DNA and protein starting from 2 weeks up to 6 months post treatment.

TG-C treatment showed improvement at 24 weeks post treatment as assessed by the primary and the secondary evaluation parameters when compared to placebo control in summary, this Phase IIb study indicated that TG-C treatment improved pain, sports activities, and quality of daily life in patients with knee osteoarthritis when compared to the placebo control.

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