enGene's mucosal gene delivery platform is successful in targeting PD-L1 expression in the gut, demonstrating effective treatment of colitis and GvHD in preclinical models

- *enGene's proprietary PD-L1 gene therapy is being developed as potential novel therapy for acute GvHD*

- *Late-breaking abstract on preclinical data from PD-L1 program will be presented at Digestive Disease Week (DDW) 2017 in Chicago*

MONTREAL, CANADA, APRIL 26, 2017 (PRNewswire) – enGene Inc., a biotechnology company developing a robust platform to deliver genes to mucosal cells lining the gut, today announced that data from preclinical studies demonstrated successful expression of programmed death-ligand 1 (PD-L1) protein in the gut showing therapeutic efficacies in mouse models of inflammatory bowel disease (IBD) and graft-versus-host disease (GvHD). This study has been selected for presentation at the DDW 2017 conference, the world's most prestigious meeting of GI professionals, attracting 15,000 physicians, researchers and academics.

"We have shown robust therapeutic response in animal models for acute GvHD and IBD, and we will focus initially on developing our PD-L1 gene therapy as a novel treatment modality to address the significant unmet medical needs in patients suffering from acute GvHD," said Dr. Anthony Cheung, CEO and President of enGene. "This program is wholly owned by enGene and it has now been on-boarded to our proprietary pipeline."

PD-L1 has been shown to play a critical role in suppressing inflammatory T-cell proliferation and promoting tolerogenic immune response. Several antibody-based inhibitors of PD-L1 have been approved as treatments for malignancies. However, PD-L1 inhibition is associated with many immune-related side effects in patients, including colitis and diarrhea. Such clinical observations suggest that PD-L1 is vital in maintaining proper immune balance in the gut and has led enGene's scientists to hypothesize that gut-localized expression of PD-L1 protein could provide a novel approach to suppress intestinal inflammation associated with IBD and GvHD.

enGene's proprietary dually-derivitized oligomeric chitosan (DDX) platform allows for packaging of plasmid DNA into nanoparticles for *in vivo* delivery to gut mucosal tissues. DNA plasmids that express a version of PD-L1 protein were formulated into nanoparticles with DDX. *In vivo* expression of PD-L1 in the colon of mice was confirmed following enema administration of the nanoparticles. In a T-cell transfer model of IBD, localized expression of PD-L1 gene in the colon achieved by enGene delivery technology led to significant therapeutic benefits. Target engagement assessment showed a significant increase in FOXP3 expression in regulatory T cells found in IBD mice treated with nanoparticles that localized PD-L1 protein expression in the colon. In addition, striking therapeutic response was noted in an acute GvHD mouse model.

enGene's late-breaking abstract presentation at DDW

Title: Successful Treatment of Murine Colitis and Acute GvHD by Gut-localized PD-L1 Expression
Date and time: May 7, 2017; 12:00 – 2:00 p.m. CDT
Location: MCP South Hall, McCormick Place
Presentation #: 2723327

About enGene
enGene Inc. is a biotechnology company developing a robust, proprietary non-viral vector platform to deliver genes to mucosal cells lining the gut. The vector system can be administered to the intestine via the oral or enema route. enGene has developed a unique gut-optimized gene delivery formulation into an orally available "Gene Pill", which has the potential to be a game-changing platform to provide oral delivery of a wide range of protein drugs. Its initial focus is enabling localized delivery of immune-modulating proteins to the gut for treating various immune disorders. enGene has established global strategic alliances with two of the world's largest pharmaceutical companies.

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