enGene Announces Positive Preliminary Phase 1/2 Data with EG-70 in BCG-Unresponsive Non-Muscle Invasive Bladder Cancer

- *83% of patients evaluable for efficacy achieved Complete Response (CR) at 3-months*
- *No Dose-Limiting Toxicities or Serious Adverse Events have been reported in the study*
- *Adverse Events (AEs) were limited to transient Grade 1 and Grade 2*

BOSTON and MONTRÉAL, Feb. 8, 2022 /PRNewswire/ - enGene Inc., a clinical-stage biotechnology company pioneering novel non-viral gene therapies for local administration into mucosal tissues enabled by its proprietary DDX platform, today announced positive results from its LEGEND study, a first-in-human Phase 1/2 clinical trial of EG-70 for the treatment of high-grade Non-Muscle Invasive Bladder Cancer (NMIBC) in patients with carcinoma in situ (CIS) that are BCG-unresponsive.

For over 30 years, the standard treatment for NMIBC has been BCG immunotherapy. However, the 60% disease recurrence and progression rates associated with BCG treatment have created an urgent unmet medical need for NMIBC patients who are left with very few options. Ultimately, the majority of NMIBC patients that do not respond to BCG will undergo radical cystectomy, a procedure in which the bladder is removed concurrently with other surrounding organs.

The results from LEGEND study to-date have shown that enGene's novel intravesical monotherapy, EG-70, is safe and well tolerated, with encouraging clinical efficacy in high-grade NMIBC patients with CIS in whom BCG has failed. Of the six patients that have reached the 3-month efficacy assessment, five have achieved complete response (CR). This marks an 83% CR rate that is trending best-in-class for an intravesical monotherapy at the end of the first treatment cycle.

"The responses observed with EG-70 are an important step towards NMIBC patients avoiding radical cystectomy," **said Dr.** Gary Steinberg**, Professor and Director at the Perlmutter Cancer Center and Department of Urology Goldstein Urology Bladder Cancer Program of NYU Langone Health**, and lead Principal Investigator for the LEGEND study. "If similar results are observed as the study progresses, I am confident that this novel intravesical monotherapy will make a meaningful impact in the lives of patients with high risk NMIBC."

"We are thrilled to announce the safety and clinical efficacy of EG-70 in patients with BCG-unresponsive NMIBC who are facing bladder removal as an alternative," **said** Jason Hanson**, Chief Executive Officer at enGene**. "EG-70 was designed ab initio as an intravesical monotherapy that would slot directly into current medical practice to provide ease-of-use and increased access to patients. With the initial results providing a clear best-in-class trajectory, we are confident that EG-70 will be the organ-sparing solution that is a game-changer for patients and physicians."

Summary of Clinical Results

- The analysis reported that 83% of patients evaluable for efficacy have achieved CR (N=6) at 3-months
- There have been no DLTs or SAEs reported to date
- AEs were limited to transient Grade 1 and Grade 2 Aes such as intermittent pain when urinating, urinary tract pain, bilateral serous otitis, increased nocturia, restlessness, and low back pain

The open label trial has a primary endpoint of safety and tolerability and a key secondary endpoint of CR rate at 3-months. With these initial data, the LEGEND study is slated to successfully meet both primary and secondary endpoints.

About enGene's DDX platform - EG-70

Based on enGene's DDX platform, EG-70 is a novel non-viral gene therapy encoding two RIG-I agonists to stimulate the innate immune system, and IL-12 to stimulate the adaptive immune system. By stimulating both arms of the immune system, intravesically administered EG-70 yields remarkable tumor regression in preclinical models of bladder cancer, with induction of effective immunological memory and minimal signs of toxicity.

About the LEGEND study

The LEGEND study, both first-in-human and first-in-class, is an open-label, monotherapy, multi-center, dose-escalation trial evaluating safety and tolerability, pharmacokinetics, pharmacodynamics, and efficacy of EG-70 administered by intravesical instillation. To learn more about the first-in-human clinical trial of EG-70 in BCG-unresponsive NMIBC, please visit ClinicalTrials.gov. For additional information about the LEGEND study, please visit thelegendstudy.com.

About enGene Inc.

enGene Inc. is a clinical-stage biotechnology company developing non-viral gene therapies based on localized delivery of nucleic acid payloads to mucosal tissues. The dually derived chitosan (DDX) platform has a high-degree of payload flexibility including DNA and various forms of RNA with broad tissue and disease applications. In preclinical models, enGene's DDX technology has been demonstrated to effectively induce expression of therapeutic genes following delivery to the lung, gastrointestinal tract and urinary bladder. Engene has developed scalable GMP-compliant manufacturing of DDX products. https://www.engene.com

Note regarding forward-looking statements

This press release contains certain "forward-looking statements" that reflect the Company's beliefs and assumptions based on currently available data and information. These forward-looking statements fall within the meaning of the "safe harbor" provisions of the U.S. Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as: "target," "believe," "expect," "will," "may," "anticipate," "estimate," "would," "positioned," "future," and other similar expressions that predict or indicate future events or trends or that are not statements of historical matters. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based only on enGene's current beliefs, expectations, and assumptions that by definition involve risks, uncertainties, that are difficult to predict and are subject to factors outside of management's control and that could cause actual results to differ substantially from statements made including but not limited to: risks associated with the success of preclinical studies, clinical trials, research and development programs, as well as regulatory approval processes. Actual results and outcomes may differ materially from those indicated in the forward-looking statements. enGene has no approved drugs available for sale marketing at this time and may never have an approved drug. You are cautioned not to rely on enGene's forward looking statements, which are only made as of the date hereof. The Company is under no obligation to update these statements.

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2/8/2022 11:07:00 AM