enGene Announces Updated Interim Results From LEGEND Pivotal Cohort

54% complete response (CR) rate at any time; 43% six month CR rate

Low rate of progression to muscle-invasive or higher disease (3.2%)

Low percentage of patients experienced treatment-related adverse events (TRAEs) (55%), nearly all of which were mild

Low percentage of patients experienced TRAEs leading to treatment interruption (2.4%) or discontinuation (2.4%)

Kaplan-Meier estimate of 12-month CR rate is 25%

enGene to host webcast to discuss results today, May 7, 2026, at 8:00 a.m. ET

BOSTON & MONTREAL - enGene Therapeutics Inc. (Nasdaq: ENGN or "enGene" or the "Company"), a clinical-stage, non-viral genetic medicines company, today reported additional interim results from the pivotal cohort of its ongoing, Phase 2 LEGEND trial of detalimogene voraplasmid (also known as detalimogene) in high-risk, Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladder cancer (NMIBC) patients with carcinoma in situ (CIS) with or without concomitant papillary disease, which enrolled 125 patients. These data, as of April 21, 2026, demonstrated that patients treated with detalimogene achieved a 54% complete response (CR) at any time and a 43% CR rate at six months. The progression rate to muscle-invasive or advanced disease was low, at 3.2%. Detalimogene was generally well tolerated with 55% of patients having experienced a treatment-related adverse event, mostly mild (Grade 1 and 2).

"These updated data continue to reinforce the favorable safety and tolerability profile of detalimogene and its clinical activity in a heavily pretreated, high-risk NMIBC patient population with limited therapeutic options. Importantly, the low rate of progression to muscle-invasive disease leaves patients eligible for other bladder-sparing therapies," said Ron Cooper, President and Chief Executive Officer, enGene. "While durability outcomes to date are not what we hoped, these data are preliminary. We are focused on evaluating the totality of the data as it evolves and plan to continue to engage with the FDA and the medical community."

LEGEND Pivotal Cohort Data Update

The interim analysis is based on the 125 patients enrolled in Cohort 1. Patients who discontinued without any disease evaluation or who discontinued after a CR were considered not evaluable for subsequent landmark CR analysis.

Efficacy overview:

- 54% (95% CI: 45%, 63%) CR at any time (67/124)
  - 91% of responses occurred at first disease assessment
- 43% (95% CI: 34%, 52%) CR rate at 6 months (52/121) with 14% (6/43) of patients having successfully converted from non-CR to CR post re-induction
- Two re-induced patients have not yet had a 6-month assessment
- Of the 52 responders at 6 months:
  - 37/44 patients who had a 9-month assessment were in CR; an additional 8 patients are pending evaluation
  - 13/22 patients who had a 12-month assessment were in CR; an additional 11 patients are pending evaluation
- A maximum of 21 patients, including the 2 currently undergoing re-induction, still have the potential to achieve a CR at 12 months
- The Kaplan-Meier (KM) estimate of the 12-month duration of response (DoR) is 25% (95% CI: 11%, 41%)
- The KM estimate for median DoR is 37.3 weeks (range: 31.6-43.9 weeks)
- Low rate of progression to muscle invasive or more advanced disease (3.2%)

Safety overview:

- 55% of patients experienced a treatment-related adverse event (TRAE)
  - Among patients with any TRAE, 91% experienced Grade 1-2 TRAEs
- Grade 3 TRAEs were reported in 6 patients (4.8%)
- Low percentage of patients experienced TRAEs leading to treatment interruption (2.4%) and treatment discontinuation (2.4%)

Among the 32 patients who had their first disease assessment after the last data analysis (October 24, 2025, reported November 11, 2025), CR rates were lower than previously reported results. The CR rate at any time was 39% and at 6 months was 32% for these patients. A preliminary subgroup analysis has not revealed any material differences in demographics or key disease characteristics. A more comprehensive analysis of these patients, including potential contributing factors, is ongoing.

As a beneficiary of FDA's RMAT designation and a participant in the FDA's CDRP program, enGene has completed the required FDA manufacturing validation batches and submitted a Statistical Analysis Plan (SAP) to the agency. The Company plans further engagement with the FDA as it approaches a potential Biologics License Application (BLA) filing and plans to provide an update in the second half of 2026.

The Company plans to share these data with the broader medical community at a Plenary Presentation at the upcoming American Urological Association meeting on May 15, 2026.

Safety Information

The overall tolerability profile associated with detalimogene is favorable. Of the 125 patients assessed for safety in Cohort 1, 69 patients (55%) experienced at least one TRAE, which were mainly Grade 1/2 in severity. The most common TRAEs were fatigue (22%), dysuria (14%), micturition urgency (12%), pollakiuria (12%), and bladder spasm (11%). Six patients experienced Grade 3 TRAEs, including one Grade 4 TRAE, which has resolved. There were no Grade 5 TRAEs reported. TRAEs leading to dose discontinuations (2.4%) and dose interruptions (2.4%) were rare.

Conference Call

enGene will host a conference call and live webcast at 8:00 a.m. ET today, May 7, 2026. Individuals interested in listening to the conference call may do so by clicking the following link, which is also available at the "Investors" section of the Company's website at www.engene.com/investors. Following the live webcast, an archived version of the call will also be available on the Company's website for 90 days.

About Non-Muscle Invasive Bladder Cancer (NMIBC)

Non-muscle invasive bladder cancer (NMIBC) is a disease that poses a significant burden on both patients and clinics and has a massive economic impact on the healthcare system. NMIBC occurs when cancer cells grow in the tissues that line the interior of the bladder, but the cancer has not yet penetrated the muscle of the bladder wall. NMIBC can present as papillary outgrowths from the bladder wall, which are typically resected, or as carcinoma in situ (CIS), which consists of flat, multifocal lesions that cannot be resected. The two forms can also co-occur. About 75%-80% of new bladder cancer diagnoses are NMIBC. Patients suffering from high-risk NMIBC who are unresponsive to the standard of care, Bacillus Calmette-Guérin (BCG), face high rates of disease recurrence (50%-70%) and are potentially subject to full removal of the bladder (cystectomy) as a curative but life-altering next step.

About Detalimogene Voraplasmid

Detalimogene is a novel, investigational, non-viral gene therapy for patients with high-risk, non-muscle invasive bladder cancer (NMIBC), including Bacillus Calmette-Guérin (BCG)-unresponsive disease. It is designed to be instilled in the bladder and elicit a powerful yet localized anti-tumor immune response.

Detalimogene was developed using the Company's Dually Derivatized Oligochitosan® (DDX) platform, a technology designed to transform how gene therapies are accessed by patients and utilized by clinicians. Medicines developed with the DDX platform can potentially overcome the limitations of viral-based gene therapies, reduce complexities related to safe handling and cold storage, and streamline both manufacturing processes and administration paradigms.

Detalimogene has received Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations from the U.S. Food and Drug Administration (FDA) based on its potential to address the high unmet medical need for patients with BCG-unresponsive carcinoma in situ (CIS) NMIBC with or without resected papillary tumors who are unable to undergo cystectomy. These designations are intended to expedite the development and review of drugs to serious or life-threatening conditions and fill an unmet medical need. Detalimogene has also been selected for the FDA's Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot (CDRP) program, designed to facilitate CMC development for therapies with compressed clinical development timeframes based on the anticipated clinical benefits of earlier patient access to the therapy.

About the LEGEND Trial

Detalimogene is being evaluated in the ongoing, open-label, multi-cohort, Phase 2 LEGEND trial to establish its safety and efficacy in high-risk NMIBC. LEGEND's pivotal cohort (Cohort 1) consists of 125 patients with high-risk, BCG-unresponsive NMIBC with CIS (with or without papillary disease) and is designed to serve as the basis of the Company's planned Biologics License Application (BLA) filing. In addition to this pivotal cohort, LEGEND includes three additional cohorts, including NMIBC patients with CIS who are naïve to treatment with BCG (Cohort 2a); NMIBC patients with CIS who have been exposed to BCG but have not received adequate BCG treatment (Cohort 2b); and BCG-unresponsive high-risk NMIBC patients with papillary-only disease (Cohort 3).

About enGene

enGene is a clinical-stage biotechnology company mainstreaming non-viral genetic medicine through the delivery of therapeutics to mucosal tissues and other organs, with the goal of creating new ways to address diseases with high clinical needs. enGene's lead program is detalimogene voraplasmid (also known as detalimogene) for patients with non-muscle invasive bladder cancer (NMIBC), a disease with a high clinical burden. Detalimogene is being evaluated in the ongoing multi-cohort LEGEND Phase 2 trial, which includes a pivotal cohort studying detalimogene in high-risk, Bacillus Calmette-Guérin (BCG)-unresponsive patients with carcinoma in situ (CIS) with or without concomitant papillary disease. Detalimogene was developed using enGene's proprietary Dually Derivatized Oligochitosan (DDX) platform, which enables penetration of mucosal tissues and delivery of a wide range of sizes and types of cargo, including DNA and various forms of RNA.

To learn more, please visit enGene.com and follow us on LinkedIn, X and BlueSky.

Forward-Looking Statements

Certain statements contained in this press release may constitute "forward-looking statements" within the meaning of the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, and "forward-looking information" within the meaning of Canadian securities laws (collectively, "forward-looking statements"). enGene's forward-looking statements include, but are not limited to, statements relating to the Company's future plans, expectations, intentions, strategies and objectives. In addition, any statements that refer to projections, forecasts or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking statements. The words "anticipate", "appear", "approximate", "believe", "continue", "could", "estimate", "expect", "foresee", "goal", "intend", "may", "might", "plan", "possible", "potential", "predict", "project", "seek", "should", "would", and similar expressions (or the negative version of such words or expressions) may identify forward-looking statements, but the absence of these words does not mean that a statement is not forward-looking. Forward-looking statements may include, for example,

statements about: detalimogene's potential efficacy, durability, safety, tolerability and ease of use profile, the development of detalimogene, the potential benefits of detalimogene, plans regarding regulatory interactions and a potential BLA submission for detalimogene, plans regarding updates on the LEGEND study, including clinical data and engagement with the FDA, and the potential benefits of medicines developed with the DDX platform. Such statements are subject to numerous important factors, risks and uncertainties, many of which are beyond enGene's control, that may cause actual events or results to differ materially from enGene's current expectations. For example, there can be no guarantee that detalimogene will successfully complete necessary clinical development phases, including achieving positive results in the pivotal cohort of the LEGEND study, or that those results or any feedback from regulatory authorities will ultimately lead to BLA submission for, and the approval of, detalimogene.

Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks, uncertainties and assumptions relating to a number of other factors, which could cause the Company's actual results, performance or achievements to differ materially from those expressed or implied by the forward-looking statements, including, without limitation, the inability of preliminary clinical data to predict the final results of the trial, changes in the results from enGene's clinical trials, including due to new data collected from the ongoing LEGEND study or future studies, subsequent analysis of existing data, and audit and verification procedures; the content and timing of decisions made by the FDA and other regulatory authorities; the Company's ability to recruit and retain qualified scientific and management personnel, establish clinical trial sites and enroll patients in its clinical trials, execute on the Company's clinical development plans; and its ability to secure regulatory approval on anticipated timelines, and other risks and uncertainties detailed in filings with Canadian securities regulators on SEDAR+ and with the U.S. Securities and Exchange Commission ("SEC") on EDGAR, including those described in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the fiscal year ended October 31, 2025 (copies of which may be obtained at www.sedarplus.ca or www.sec.gov).

You should not place undue reliance on any forward-looking statements, which speak only as of the date on which they are made. enGene anticipates that subsequent events and developments will cause enGene's assessments to change. While enGene may elect to update these forward-looking statements at some point in the future, enGene specifically disclaims any obligation to do so, unless required by applicable law. Nothing in this press release should be regarded as a representation by any person that the forward-looking statements set forth herein will be achieved or that any of the contemplated results of such forward-looking statements will be achieved.

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