enGene to Present Three Posters at the 2025 ASCO Genitourinary Cancers Symposium (ASCO GU) Highlighting the Ongoing Clinical Development of Detalimogene Voraplasmid for the Treatment of Non-Muscle Invasive Bladder Cancer (NMIBC)

Detalimogene voraplasmid is a novel, investigational, pivotal-stage, non-viral genetic medicine designed to mitigate systemic risks and deliver durable efficacy for patients with high-risk NMIBC, including Bacillus Calmette-Guérin (BCG)-unresponsive disease

Ongoing, pivotal, registrational LEGEND study is actively enrolling patients with sites participating in the USA, Canada, Europe and the Asia-Pacific region

enGene's Dually Derivatized Oligochitosan® (DDX) platform is designed to potentially overcome the limitations of viral gene therapies and increase patient and clinician access to therapies

BOSTON & MONTREAL - enGene Holdings Inc. (Nasdaq: ENGN, or "enGene" or the "Company"), a clinical-stage genetic medicines company whose non-viral lead investigational product detalimogene voraplasmid (also known as detalimogene, and previously EG-70) is in an ongoing pivotal study in patients with high-risk, Bacillus Calmette-Guérin (BCG)-unresponsive, non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS), today announced three poster presentations at the 2025 ASCO Genitourinary Cancers Symposium (ASCO GU) to be held February 13-15, 2025 in San Francisco.

"Patients with NMIBC often face a debilitating, multi-year journey with limited treatment options and significant negative impacts to daily life. These patients are most commonly treated by community urologists who face unique challenges accessing and providing optimal care, including global treatment shortages and complex usage and handling," said Ron Cooper, Chief Executive Officer of enGene. "We are determined to develop genetic therapies for underserved conditions like NMIBC and ultimately provide a long overdue treatment innovation designed to advance care and ease the treatment burden."

There are an estimated 730,000 patients in the U.S. living with bladder cancer, and approximately 75% to 80% of all bladder cancer diagnoses are NMIBC. Among patients diagnosed with high-risk NMIBC, more than half (50-70%) will become BCG-unresponsive and experience recurrence and/or progression after treatment.

"The current treatment options for patients with high-risk NMIBC are sadly sub-optimal, often resulting in disease progression, recurrence or removal of the bladder as a life-altering measure of last resort," said Anthony Cheung, Chief Scientific Officer and Co-Founder of enGene. "If approved, we believe detalimogene voraplasmid will be uniquely compelling to both patients and physicians by combining a streamlined administration process designed to ease the treatment experience with durable efficacy and a favorable safety and tolerability profile."

Presentation details are outlined below:

Preliminary results from LEGEND: A phase 2 study of detalimogene voraplasmid (EG-70), a novel, non-viral intravesical gene therapy for patients with BCG-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS)

- **Abstract #:** 802
- **Poster #:** F29
- **Clinical Trial Registration Number:** NCT04752722
- **Session Title:** Poster Session B: Urothelial Carcinoma
- **Presentation Date/Time:** Friday, February 14 from 11:30am-12:45pm PT
- **Location:** Level 1, West Hall
- **Presenting Author:** John Taylor III, MD, MS, Professor of Urology & Cancer Biology, University of Kansas

Cancer Center

Mechanism of action and translation to the clinic of detalimogene voraplasmid (EG-70): A novel, investigational, non-viral immunotherapy for non-muscle-invasive bladder cancer (NMIBC)

- **Abstract #:** 826
- **Poster #:** G22
- **Clinical Trial Registration Number:** NCT04752722
- **Session Title:** Poster Session B: Urothelial Carcinoma
- **Presentation Date/Time:** Friday, February 14 from 11:30am-12:45pm PT
- **Location:** Level 1, West Hall
- **Presenting Author:** Vikram Narayan, MD, Assistant Professor in the Department of Urology at Emory University School of Medicine

A phase 1/2 study of detalimogene voraplasmid (EG-70) intravesical monotherapy for patients with high-risk non-muscle invasive bladder cancer (NMIBC)

- **Abstract #:** TPS886
- **Poster #:** J14
- **Clinical Trial Registration Number:** NCT04752722
- **Session Title:** Trials In Progress Poster Session B: Urothelial Carcinoma
- **Presentation Date/Time:** Friday, February 14 from 11:30am-12:45pm PT
- **Location:** Level 1, West Hall
- **Presenter:** Shreyas Joshi, MD, MPH, Assistant Professor in the Department of Urology at Emory University School of Medicine

About Non-Muscle Invasive Bladder Cancer (NMIBC)

Non-muscle invasive bladder cancer (NMIBC) is a disease with a significant patient burden, high clinical needs and massive economic impact on our 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 deeper into the muscle of the bladder wall. 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 subject to full removal of the bladder (cystectomy) as a curative but life-altering next step.

About Detalimogene Voraplasmid

Detalimogene voraplasmid (formerly known as EG-70) is a novel, investigational, non-viral genetic medicine 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 voraplasmid has received Fast Track designation from the U.S. Food and Drug Administration (FDA) based on its potential to address a 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. Fast Track designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need.

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, simplify safe handling and cold storage complexities and streamline both manufacturing processes and administration paradigms.

About the Pivotal LEGEND Trial

Detalimogene voraplasmid is being evaluated in the ongoing, open-label, multi-cohort, Phase 2 LEGEND trial to establish its safety and efficacy in high-risk, non-muscle invasive bladder cancer (NMIBC). LEGEND's pivotal cohort (Cohort 1) consists of approximately 100 patients with high-risk, Bacillus Calmette-Guérin (BCG)-unresponsive NMIBC with carcinoma in situ (CIS) (with or without papillary disease) and is designed to serve as the basis of the Company's Biologics License Application (BLA) filing. In addition to this pivotal cohort, three additional cohorts are actively enrolling patients, 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).

The LEGEND trial is actively enrolling patients with sites participating in the USA, Canada, Europe and the Asia-Pacific region. For more information, please visit TheLegendStudy.com.

About enGene

enGene is a clinical-stage biotechnology company mainstreaming genetic medicines 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 mission is to deliver innovative non-viral medicines to improve the quality of life for people with urological cancers. enGene's lead program is detalimogene voraplasmid for patients with non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) - a disease with a high clinical burden - who are unresponsive or naïve to treatment with Bacillus Calmette-Guérin (BCG).

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

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 regarding enGene's management teams' expectations, hopes, beliefs, intentions, goals or strategies regarding the future. 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", "intends", "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: our beliefs as to the potential benefits of detalimogene, including its potential to provide a highly differentiated treatment option, if approved, and the potential benefits of medicines developed with the DDX platform.

Many factors, risks, uncertainties and assumptions 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 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 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, 2024 (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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20250129906939/en/

Media Contact:
Green Room Communications
908-577-4531
mediainquiries@grcomms.com

Investor Contact: investors@engene.com

1/30/2025 8:00:00 AM