enGene Reports First Quarter 2025 Financial Results and Provides Business Update

Additional preliminary data from ongoing LEGEND study of detalimogene in BCG-unresponsive NMIBC with CIS, including pivotal cohort, anticipated in 2H 2025

Biologics License Application (BLA) filing planned for mid-2026 remains on track

Cash and marketable securities of $272.8 million expected to provide runway into 2027

BOSTON & MONTREAL - enGene Holdings Inc. (Nasdaq: ENGN, or "enGene" or the "Company"), a clinical-stage, non-viral genetic medicines company, today announced its financial results for the first quarter ended January 31, 2025, and provided a business update.

"With the expansion of LEGEND study sites into Europe and Asia, enrollment in our pivotal cohort continues to track in-line with our plan to file a BLA for detalimogene in mid-2026," said Ron Cooper, Chief Executive Officer of enGene. "We look forward to the remainder of 2025, during which we expect to announce enrollment completion of LEGEND's pivotal cohort and provide additional updates on other LEGEND study cohorts. We continue to believe in detalimogene's opportunity to transform the treatment landscape of NMIBC through a unique and differentiated overall profile that is highly attractive to both patients and physicians due to its potential for tolerability, ease-of-use, and efficacy."

Recent Corporate Updates

LEGEND study trial site expansion: Over the course of the first quarter of 2025, the Company expanded its clinical footprint for the LEGEND study with the addition of trial sites in Europe and Asia. In addition, all four of LEGEND's cohorts are now open for enrollment, including the pivotal cohort evaluating detalimogene voraplasmid (also known as detalimogene, and previously EG-70) in high-risk, BCG-unresponsive non-muscle invasive bladder cancer (NMIBC) with CIS as well as Cohort 2a and 2b (i.e., NMIBC patients with CIS who are naïve to treatment with BCG and NMIBC patients with CIS who have been exposed to BCG, but have not received adequate BCG treatment), and Cohort 3 (BCG-unresponsive high-risk NMIBC patients with papillary-only disease). Global enrollment is taking place under an amended protocol that is designed to align with the American Urological Association's current treatment guidelines and real-world practices.

LEGEND study now includes maintenance dosing: The amended LEGEND protocol allows patients from all cohorts who are in complete response at 12 months to continue receiving detalimogene on a dose-reduced maintenance schedule throughout their second year of treatment. At the end of year two, patients may elect to remain on maintenance therapy for another year, for a total of three years of therapy. Maintenance treatment consists of two instillations of detalimogene per three-month cycle, administered at week one and at week two of each cycle. The maintenance regimen is intended to evaluate the potential of longer-term detalimogene treatment to improve or maintain durability of response with the lower patient burden associated with a less frequent dosing regimen.

First Quarter 2025 Financial Results

As of January 31, 2025, cash, cash equivalents and marketable securities were $272.8 million. The Company expects that its existing cash, cash equivalents and marketable securities will fund operating expenses, debt obligations and capital expenditures into 2027.

Three Months ended January 31, 2025

Total operating expenses were $26.6 million for the three months ended January 31, 2025, compared to $10.8 million for the three months ended January 31, 2024. Research and development expenses increased by $14.3 million, mainly due

to increasing manufacturing and clinical costs related to our pivotal LEGEND study and headcount costs. General and administrative expenses increased by $1.5 million, primarily driven by increased headcount intended to support the Company's operation as a publicly traded company.

For the three months ended January 31, 2025, net loss attributable to common shareholders was approximately $24.6 million, or $0.48 per share, compared to approximately $10.7 million, or $0.46 per share, for the same period for the three months ended January 31, 2024. The increase in net loss is mainly attributed to the increase in operating expenses, partially offset by net interest income earned during the period.

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

Detalimogene 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 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.

Detalimogene has received Fast Track designation 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. 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.

About the Pivotal 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, 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 planned 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.

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 lead program is 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 study, which includes a registrational cohort studying detalimogene in Bacillus Calmette-Guérin (BCG)-unresponsive patients with carcinoma in situ (CIS). 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. For more information, visit enGene.com.

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 plans regarding the timing of our planned BLA submission to the Food and Drug Administration, our expectations as to the timing and anticipated results of the LEGEND study, including the timing of preliminary data or other updates, our expectations regarding completion of enrollment in the LEGEND study, including the timing, our expectations regarding the potential impact of the amendment of the LEGEND study protocol, the potential benefits of detalimogene, including its potential impact on the treatment landscape and attractiveness to patients and physicians, the potential benefits of medicines developed with the DDX platform and the expected period over which we estimate our cash, cash equivalents and marketable securities will be sufficient to fund our current operating plan.

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.

enGene Holdings Inc.
Condensed Consolidated Statements of Operations Information
(unaudited)
(Amounts in thousands of USD, except share and per share data)

	Three Months Ended January 31,	
	2025	**2024**
Operating expenses:		
Research and development	$ 19,974	$ 5,638
General and administrative	6,639	5,135
Total operating expenses	26,613	10,773
Loss from operations	26,613	10,773
Total other (income) expense, net	(2,015)	(32)
Net loss before income tax	24,598	10,741
Provision for (recovery of) income tax	18	(30)
Net loss	$ 24,616	$ 10,711
Other comprehensive loss:		
Unrealized gain on available-for-sale investments	$ (144)	$ -
Total comprehensive loss	$ 24,472	$ 10,711
Net loss attributable to common shareholders, basic and diluted	24,616	10,711
Weighted-average common shares outstanding, basic and diluted	50,976,958	23,197,976
Net loss per share of common shares, basic and diluted	$ 0.48	$ 0.46

enGene Holdings Inc.
Condensed Consolidated Balance Sheet Information
(unaudited)
(Amounts in thousands of USD)

	January 31, 2025	**October 31, 2024**
Cash, cash equivalents and marketable securities	$ 272,792	$ 297,859
Total assets	285,932	311,173
Total liabilities	35,982	38,561
Total shareholders' equity	249,950	272,612

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