enGene Reports Second Quarter 2025 Financial Results and Provides Business Update

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

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

EMA Scientific Advice completed; Indicated data could be suitable for a Contingent Marketing Authorization Application

Cash and marketable securities of $251.5 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 second quarter ended April 30, 2025, and provided a business update.

"We have seen strong enrollment in the pivotal cohort of our LEGEND study," said Ron Cooper, Chief Executive Officer of enGene. "This positions us to stay on track for our planned trial updates across all cohorts in the second half of 2025 and a potential BLA filing in mid-2026, advancing our goal to introduce a novel, non-viral therapy that could redefine treatment for patients with high-risk non-muscle invasive bladder cancer."

Recent Corporate Updates

Key executive hires and management appointments: In May 2025, the Company announced the appointment of Amy Pott as Chief Global Commercialization Officer. Ms. Pott joined enGene from Astellas Pharma, where she most recently served as Senior Vice President, Strategic Brand Marketing, Ophthalmics and Rare Diseases, and previously as Head of Commercial, Gene Therapies. She will serve as the Company's first dedicated executive for commercialization planning and execution.

LEGEND study enrollment update: Over the course of the first and second quarters of 2025, the Company expanded its clinical footprint for the LEGEND study with the addition of trial sites in Europe and Asia. The pivotal cohort evaluating detalimogene voraplasmid (also known as detalimogene, and previously EG-70) in high-risk, Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) remains on track for our planned BLA in mid-2026. The Company expects to provide an update from LEGEND's pivotal cohort in the second half of 2025.

European Medicines Agency (EMA) Scientific Advice: The Company shared detalimogene preclinical and clinical data through the EMA Scientific Advice process. During the dialogue, EMA indicated that it broadly agrees that these data could be suitable for a Conditional Marketing Authorization Application (CMA) submission for detalimogene in BCG-unresponsive NMIBC with CIS assuming a positive benefit-risk ratio.

BIOTECanada Gold Leaf Biotech Company of the Year Award Winner: The Biotech Company of the Year Award recognizes a company that is transforming the future of healthcare through groundbreaking advancements. The Company is proud and honored to be recognized with this distinction.

Second Quarter 2025 Financial Results

As of April 30, 2025, cash, cash equivalents and marketable securities were $251.5 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 April 30, 2025

Total operating expenses were $27.1 million for the three months ended April 30, 2025, compared to $17.3 million for the three months ended April 30, 2024. Research and development expenses increased by $10.4 million, mainly due to increasing manufacturing and clinical costs related to our pivotal LEGEND study and personnel-related costs. General and administrative expenses decreased by $0.5 million, primarily driven by decreased reliance on professional services to support the Company's operation as a publicly traded company.

For the three months ended April 30, 2025, net loss attributable to common shareholders was approximately $25.8 million, or $0.51 per share, compared to approximately $15.0 million, or $0.38 per share, for the same period for the three months ended April 30, 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 that poses a significant burden on both patients and clinics and has a 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 the muscle of the bladder wall. NMIBC can take the form of papillary outgrowths from the bladder wall, which are typically resected, or carcinoma in situ (CIS), flat, multifocal lesions that are unable to be resected, and the two can 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 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, NMIBC, including 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 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, NMIBC. LEGEND's pivotal cohort (Cohort 1) consists of approximately 100 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). 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 pivotal 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 a potential CMA submission to the EMA, our expectations regarding completion of enrollment in the LEGEND study, including the timing, 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 April 30,		Six Months Ended April 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$20,209	$9,855	$40,183	$15,493
General and administrative	6,915	7,455	13,554	12,590
Total operating expenses	27,124	17,310	53,737	28,083
Loss from operations	27,124	17,310	53,737	28,083
Total other (income) expense, net	(1,549)	(2,347)	(3,564)	(2,379)
Net loss before income tax	25,575	14,963	50,431	25,704
Provision for (recovery of) income tax	240	21	258	(9)
Net loss	$25,815	$14,984	$50,431	$25,695
Other comprehensive loss:				
Unrealized gain on available-for-sale investments	$(306)	$-	$(450)	$-
Total comprehensive loss	$25,509	$14,984	$49,981	$25,695
Net loss attributable to common shareholders, basic and diluted	25,815	14,984	50,431	25,695
Weighted-average common shares outstanding, basic and diluted	51,019,363	39,443,768	50,997,987	31,186,238
Net loss per share of common shares, basic and diluted	$0.51	$0.38	$0.99	$0.82

enGene Holdings Inc.

Condensed Consolidated Balance Sheet Information

(unaudited)

(Amounts in thousands of USD)

	April 30, 2025	October 31, 2024
Cash, cash equivalents and marketable securities	$251,501	$297,859
Total assets	266,651	311,173
Total liabilities	39,360	38,561
Total shareholders' equity	227,291	272,612

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