enGene Reports Third Quarter 2025 Financial Results and Provides Business Update

Achieved target enrollment milestone for LEGEND trial pivotal cohort in high-risk BCG unresponsive NMIBC with carcinoma in situ

LEGEND's pivotal cohort data update planned for 4Q 2025

Detalimogene granted Regenerative Medicine Advanced Therapy (RMAT) designation by FDA

Biologic License Application (BLA) submission planned for 2H 2026

Cash and marketable securities of $224.9 million expected to provide runway into 2027

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

"Reaching target enrollment in LEGEND's pivotal Cohort 1 and securing RMAT designation are important milestones that mark our continued momentum," said Ron Cooper, Chief Executive Officer of enGene. "We look forward to providing a data update from the LEGEND pivotal cohort later this year. These advances bring us closer to our planned BLA filing in 2026 with the ultimate goal of delivering detalimogene to patients as a therapy designed for efficacy, safety, and ease-of-use."

Recent Corporate Updates

LEGEND study enrollment update: The Company announced that it achieved its target enrollment milestone of 100 patients with high-risk, BCG-unresponsive NMIBC carcinoma in-situ (CIS) with or without concomitant papillary disease for the pivotal cohort of its ongoing, open-label, multi-cohort Phase 2 LEGEND trial of detalimogene. Patients in the screening process remain eligible for potential enrollment in Cohort 1.

Detalimogene granted RMAT designation: The Company announced that the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation to detalimogene voraplasmid for the treatment of high-risk (HR), Bacillus Calmette-Guérin (BCG)-unresponsive, non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) based on previously disclosed data from the ongoing LEGEND trial. This designation provides enGene with several regulatory advantages, including early and frequent engagement with the FDA and the potential for rolling submission and priority review. Detalimogene was also granted Fast Track designation from the FDA in November 2020.

Key board and management appointments: The Company announced the appointment of Philip Astley-Sparke, William Grossman, M.D., Ph.D., and Michael Heffernan R.Ph., to its Board of Directors, and the promotion of Matthew Boyd to Chief Regulatory Officer, Jill Buck to Chief Development Officer, and Katherine Chan, M.D., M.P.H. to Executive Director, Urology Clinical Lead. Their combined experience across gene therapy, oncology, urology, clinical development, and global product launches will support enGene's strategic transition toward its planned commercialization of detalimogene.

Anticipated Milestones

- Updated preliminary data from LEGEND trial's pivotal cohort in 4Q 2025.
- Trial in progress updates from LEGEND's additional cohorts, including HR-NMIBC patients with CIS who are naïve to treatment with BCG (Cohort 2a); HR-NMIBC patients with CIS who have been exposed to BCG but have not received adequate BCG treatment (Cohort 2b); and BCG-unresponsive HR-NMIBC patients with papillary-only disease (Cohort 3), in 4Q 2025.

- Planned BLA filing for LEGEND's pivotal cohort in 2H 2026.

Third Quarter 2025 Financial Results

As of July 31, 2025, cash, cash equivalents and marketable securities were $224.9 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 July 31, 2025

Total operating expenses were $29.9 million for the three months ended July 31, 2025, compared to $16.8 million for the three months ended July 31, 2024. Research and development expenses increased by $11.0 million, mainly due to increasing manufacturing and clinical costs related to the pivotal cohort of our LEGEND study and personnel-related costs. General and administrative expenses increased by $2.2 million, primarily driven by increased personnel-related expense to support the operation of a public company and increased reliance on professional services to support the Company's preparations for potential commercialization.

For the three months ended July 31, 2025, net loss attributable to common shareholders was approximately $28.9 million, or $0.57 per share, compared to approximately $14.1 million, or $0.32 per share, for the same period for the three months ended July 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 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 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. The RMAT program is intended to expedite the development and review of regenerative medicine therapies for serious or life-threatening conditions, where preliminary clinical evidence suggests potential to address unmet medical needs. Similarly, 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 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 gene therapy 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, and previously EG-70) 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 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: the Company's expectations as to the timing and anticipated results of the LEGEND study, including the timing of preliminary data or other updates, the Company's expectations regarding the timing of the planned BLA submission to the Food and Drug Administration, the Company's expectations regarding over-enrollment in the pivotal cohort of the LEGEND study, the Company's expectations regarding the RMAT designation and its impact on the development and/or regulatory review of detalimogene, the potential benefits of detalimogene, the potential benefits of medicines developed with the DDX platform, and the expected period over which the Company estimates its cash 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 July 31,		Nine Months Ended July 31,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 22,575	$ 11,549	$ 62,758	$ 27,042
General and administrative	7,372	5,210	20,926	17,800
Total operating expenses	29,947	16,759	83,684	44,842
Loss from operations	29,947	16,759	83,684	44,842
Total other expense (income), net	(1,195)	(2,582)	(4,759)	(4,961)
Net loss before income tax	28,752	14,177	78,925	39,881
Provision for (recovery of) income tax	239	(29)	497	(38)
Net loss	$ 28,991	$ 14,148	$ 79,422	$ 39,843
Other comprehensive loss:				
Unrealized gain on available-for-sale investments	$ 240	$ -	$ (210)	$ -
Total comprehensive loss	$ 29,231	$ 14,148	$ 79,212	$ 39,843
Net loss attributable to common shareholders, basic and diluted	28,991	14,148	79,422	39,843
Weighted-average common shares outstanding, basic and diluted	51,097,711	44,168,986	51,031,618	35,564,767
Net loss per share of common shares, basic and diluted	$ 0.57	$ 0.32	$ 1.56	$ 1.12

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

	July 31, 2025	October 31, 2024
Cash, cash equivalents and marketable securities	$ 224,924	$ 297,859
Total assets	245,550	311,173
Total liabilities	44,878	38,561
Total shareholders' equity	200,672	272,612

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