enGene Reports Full Year 2025 Financial Results and Provides Business Update

Completed enrollment in LEGEND's pivotal cohort with 125 patients in high-risk, BCG-unresponsive NMIBC with carcinoma in situ

Reported preliminary 6-month complete response rate (62%) in pivotal cohort patients enrolled under amended LEGEND protocol

enGene selected for FDA's CDRP Program to support CMC readiness

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

Cash and marketable securities plus proceeds from November financing expected to provide cash runway into 2H 2028

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

"We are closing 2025 in a position of strength: enrollment in LEGEND's pivotal cohort is complete and the recent data update from the LEGEND pivotal cohort supports an emerging, competitive profile for the use of detalimogene as the first choice therapy in BCG-unresponsive NMIBC if approved," said Ron Cooper, President and Chief Executive Officer. "With recent acceptance into FDA's CDRP program and a cash position of $342.4 million, extending our runway into the second half of 2028, we are entering the new year laser-focused on scaling our organization in preparation for our planned BLA filing in the second half of 2026 and a potential approval in 2027."

Recent Corporate Updates

LEGEND pivotal cohort update: In November, the Company reported updated preliminary data from LEGEND's pivotal cohort for patients who were enrolled following a protocol amendment that was implemented in the fourth quarter of 2024 ("post-amendment patients"). The preliminary analysis of the post-amendment patients with at least one post-baseline disease assessment included 62 patients at 3 months and 37 patients at 6 months. Data from these patients demonstrated:

- 63% complete response (CR) rate at any time (n=62);
- 56% CR rate at 3 months (n=62);
- 62% CR rate at 6 months (n=37), including 4 patients who successfully converted to CR post reinduction; and
- All 5 patients who completed the 9-month assessment had a CR.

The Company completed enrollment of 125 patients in the pivotal cohort, exceeding its target by 25%. Data from these patients demonstrated a generally favorable tolerability profile:

- 42% of patients experienced a treatment-related adverse event (TRAE);
- 1.6% of patients experienced dose interruptions due to TRAEs; and
- 0.8% of patients experienced dose discontinuations due to TRAEs.

The Company continues to believe that detalimogene's emerging profile supports its potential first line use in patients with high-risk, BCG-unresponsive non-muscle invasive bladder cancer with carcinoma in situ (CIS).

Additional LEGEND cohort updates: In conjunction with its November data update for LEGEND's pivotal cohort, the Company provided an enrollment update for LEGEND's three additional cohorts:

- Cohort 2a, evaluating detalimogene in patients with high-risk NMIBC with CIS who are naïve to treatment with BCG, has enrolled 30 patients.
- Cohort 2b, evaluating detalimogene in patients with high-risk, NMIBC with CIS who have been exposed to BCG

but have not received adequate BCG treatment, has enrolled 45 patients.
- Cohort 3, evaluating detalimogene in BCG-unresponsive patients with high-risk, papillary-only NMIBC, has enrolled 36 patients.

Successful completion of public offering: In November, the Company successfully closed an underwritten public offering of its common shares and pre-funded warrants at an offering price of $8.50 per share and $8.4999 per pre-funded warrant. The offering totaled $140.1 million in net proceeds, following the full exercise of the underwriters' option to purchase additional shares.

Development and Readiness Pilot (CDRP) Program: In November, the Company was selected as one of nine companies to participate in FDA's Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot (CDRP) program. The FDA created the CDRP Program to facilitate CMC development for therapies with compressed clinical development timeframes based on the anticipated clinical benefits of earlier patient access to the therapy. The initiative is designed to promote earlier and more structured engagement between sponsors and the FDA on CMC development strategies, and since its inception, has led to increased collaboration with the FDA so sponsors can confidently scale up manufacturing capacity while clinical development is ongoing. Acceptance into the program further supports enGene's confidence in detalimogene's potential and aligns with enGene's commitment to strong manufacturing practices as clinical development advances.

Key executive hire: In September, enGene announced the appointment of Hussein Sweiti, M.D., MSc, as Chief Medical Officer. Dr. Sweiti is a surgical oncologist and physician-scientist with more than 15 years of experience spanning clinical practice, oncology clinical research, global drug development, regulatory submissions, and medical affairs. He most recently served as Global Medical Head, Oncology Clinical Development at Johnson & Johnson (J&J), where he led end-to-end clinical strategy and execution for the company's bladder cancer portfolio. He was intimately involved in U.S. Food and Drug Administration (FDA) interactions that culminated in J&J's FDA approval in high-risk, BCG-unresponsive non-muscle invasive bladder cancer (NMIBC) earlier this month.

Anticipated Milestones

- Following discussion with the FDA on its statistical analysis plan and the accumulation of sufficient 12-month CR data points, enGene expects to provide a data update on the LEGEND trial's pivotal cohort in the second half of 2026.
- Planned BLA filing for LEGEND's pivotal cohort in the second half of 2026.

Fourth Quarter 2025 Financial Results

As of October 31, 2025, cash, cash equivalents and marketable securities were $202.3 million. An additional $140.1 million in net proceeds was raised during a public offering in November. The Company expects that its cash and cash equivalents together with the net proceeds from the November financing will fund operating expenses, debt obligations and capital expenditures into the second half of 2028.

Full Year Financial Results ended October 31, 2025

Total operating expenses were $123.2 million for the full year ended October 31, 2025, compared to $62.3 million for 2024. Research and development expenses increased by $56.2 million, primarily driven by increased manufacturing, personnel and clinical costs related to our LEGEND trial and in preparation for our planned Biologics License Application submission in the second half of 2026. General and administrative expenses increased by $4.7 million, primarily driven by personnel and facilities costs as the Company scales its general and administrative function to support the operation of a public company.

For the full year ended October 31, 2025, net loss attributable to common shareholders was approximately $117.3 million, or $2.29 per share, compared to approximately $55.1 million, or $1.46 per share, for the full year 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. Detalimogene has also been selected to participate in the FDA's Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot (CDRP) program. The FDA created the CDRP Program 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). 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 team's 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", "goal", "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 expected period over which enGene estimates its cash and marketable securities will be sufficient to fund its current operating plan, the CDRP program and its potential benefits, the future development of detalimogene, the potential benefits of detalimogene, including its ability to become a first line therapy in BCG-unresponsive NMIBC and its attractiveness to urologists, plans regarding regulatory interactions and potential BLA submission for detalimogene, 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 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.

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

	Year Ended October 31,	
	2025	**2024**
Operating expenses:		
Research and development	$ 94,480	$ 38,315
General and administrative	28,685	23,982
Total operating expenses	123,165	62,297
Loss from operations	123,165	62,297
Total other expense (income), net	(5,863)	(7,136)
Net loss before income tax	117,302	55,161
Provision for (recovery of) income tax	-	(19)
Net loss	$ 117,302	$ 55,142
Other comprehensive loss:		
Unrealized gain on available-for-sale investments	$ (531)	$ (403)
Total comprehensive loss	$ 116,771	$ 54,739
Net loss attributable to common shareholders, basic and diluted	117,302	55,142
Weighted-average common shares outstanding, basic and diluted	51,119,479	37,782,346
Net loss per share of common shares, basic and diluted	$ 2.29	$ 1.46

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

	October 31, 2025	**October 31, 2024**
Cash, cash equivalents and marketable securities	$ 202,258	$ 297,859
Total assets	221,468	311,173
Total liabilities	53,758	38,561
Total shareholders' equity	167,710	272,612

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