enGene's Detalimogene Selected for FDA Manufacturing Pilot Program to Support Manufacturing Readiness

enGene one of nine companies awarded CDRP per year

Program aims to support CMC development of products with expedited clinical development timeframes and provide patients with earlier access to these products

Detalimogene has already been manufactured at commercial scale, and CDRP provides additional FDA CMC interactions ahead of a potential BLA submission in 2H 2026

BOSTON & MONTREAL - enGene Holdings Inc. (Nasdaq: ENGN or "enGene" or the "Company"), a clinical-stage, non-viral genetic medicines company, today announced that the U.S. Food and Drug Administration (FDA) has selected detalimogene voraplasmid (also known as detalimogene and previously EG-70) to participate in the Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot (CDRP) Program. Detalimogene is a novel, investigational, non-viral gene therapy for patients with high-risk, non-muscle invasive bladder cancer (NMIBC).

"Manufacturing readiness in drug development is often underappreciated. We have already scaled detalimogene manufacturing to commercial-level, and CDRP is expected to help ensure CMC readiness for filing and commercialization," said Ron Cooper, President and Chief Executive Officer. "Our progress on the manufacturing front is in sync with detalimogene clinical development, where we recently announced improved six-month complete response rate in the LEGEND trial pivotal cohort."

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 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.

Last month, enGene reported positive preliminary results from the LEGEND trial's pivotal cohort, studying detalimogene in patients with high-risk, BCG-unresponsive NMIBC with carcinoma in situ (CIS) with or without concomitant papillary disease. Results showed an improved complete response (CR) rate of 62% at six months. Safety data from 125 patients demonstrated a favorable tolerability profile, with low rates of treatment-related adverse events (42%) and dose interruptions (1.6%).

Detalimogene has received Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations from the FDA. The Company plans to submit a Biologics License Application (BLA) for detalimogene in the second half of 2026.

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 the 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 the 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 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 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 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 CDRP program and its potential benefits, the development of detalimogene, the potential benefits of detalimogene, 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, 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.

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