FDA Grants RMAT Designation for enGene's Detalimogene, Enabling Potential for Expedited Review in High-Risk, Non-Muscle Invasive Bladder Cancer

Regenerative Medicine Advanced Therapy (RMAT) designation provides access to accelerated development tools

Designation recognizes the potential of detalimogene to address significant unmet need

BOSTON & MONTREAL - enGene Holdings Inc. (Nasdaq: ENGN, or "enGene" or the "Company"), a clinical-stage, non-viral gene-based immunotherapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to detalimogene voraplasmid (also known as detalimogene, and previously EG-70), the Company's lead investigational therapy for the treatment of high-risk, Bacillus Calmette-Guérin (BCG)-unresponsive, non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS).

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. This designation provides enGene with several regulatory advantages, including early and frequent engagement with the FDA, potential for rolling review and priority review, and other benefits like Fast Track and Breakthrough Therapy designations.

"Receiving the RMAT designation highlights the promising profile of detalimogene and its potential to address the high unmet need in NMIBC," stated Ron Cooper, Chief Executive Officer of enGene. "Bladder cancer patients with limited options cannot wait, and we are enthusiastic about potentially expediting the regulatory process to bring a first-in-class treatment to patients."

The designation was based on previously disclosed preliminary results from the ongoing pivotal LEGEND study, which demonstrated compelling clinical activity and a generally favorable tolerability profile in patients with BCG-unresponsive NMIBC with CIS.

Detalimogene is designed for streamlined administration in urology clinics - including community practices, where approximately 70% of urologists provide care. The therapy is being evaluated for its ability to treat NMIBC though the non-viral stimulation of a local immune response in the bladder, presenting a potentially transformative option for patients who otherwise face limited choices beyond radical cystectomy.

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 gene-based immunotherapy for patients with high-risk 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 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 expectations regarding the RMAT designation and its impact on the development and/or regulatory review of detalimogene, the potential benefits of detalimogene, including its potential impact on the treatment landscape and attractiveness to patients and physicians, and the potential benefits of medicines developed with the DDX platform.

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.

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