MeiraGTx Announces Poster Presentation at the 2023 American Society of Gene and Cell Therapy (ASGCT) Spotlight on Immuno-Oncology

August 01, 2023

RiboCAR-T cell activity can be precisely tuned and "remotely" controlled to improve the efficacy, durability and safety of CAR-T cell therapy

LONDON and NEW YORK, Aug. 01, 2023 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced a poster presentation at the 2023 American Society of Gene and Cell Therapy (ASGCT) Spotlight on Immuno-oncology, which is being held from August 1-2, 2023, in Seattle, WA.

"We are pleased to share data at this year's ASGCT Spotlight on Immuno-oncology on our RiboCAR technology, a synthetic riboswitch-based gene regulation system with the potential to transform CAR-T cell therapy," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "This innovative approach allows us to precisely and reversibly control the expression of CAR in a dose response to novel small molecule inducers, from undetectable at baseline to at least as high as levels of consitutively expressed CAR driven by the small molecule dose. This degree of control of timing and level of CAR expression is unprecedented."

Dr. Forbes continued, "The impact of precise CAR control appears to be profound. We have demonstrated impressive improvement in CAR-T activity, cell differentiation state and durability in response to regulated RiboCAR expression as well as providing a much needed safety switch. We believe this is a game changing technology with the potential to have meaningful impact on outcomes for patients treated with CAR-T therapy."

The poster will be available on the Posters and Publications page of the Company's website on August 1, 2023.

The details of the poster presentation are as follows:

Poster Number: 21
Abstract Title: *Riboswitch-regulated Chimeric Antigen Receptor (RiboCAR) Enhances CAR-T cell Anti-cancer Efficacy*
Date: August 1-2, 2023
Time: 7:00am - 10:00am
Room: Emerald Ballroom

Chimeric antigen receptor (CAR)-T cell therapy is a promising treatment for certain cancers. However, it is increasingly evident that the level and timing of CAR molecule expression is important for CAR-T cell activation, durability and anti-cancer activities. Here, we present the development of RiboCAR, a system for precise control of CAR expression via orally available small molecule inducers. Unlike previously reported regulatable CAR platforms that utilize viral protease or chemical-induced protein dimerization, RiboCAR contains a synthetic mammalian ON riboswitch in the coding sequence of the CAR transgene, in which the aptamer functions as a sensor for a specific novel small molecule inducer. The expression level of the CAR gene is precisely dependent on the level of the riboswitch inducer. CAR is undetectable in the absence of the small molecule, and a precise dose response in CAR levels is achieved with increasing dose of small molecule, reaching levels higher than constitutively active CAR upon maximal small molecule dose. Induced CAR expression diminishes following withdrawal of the small molecule. Consistent with small molecule induced expression of the CAR molecule, we controlled CAR-T cell activity by the small molecule inducer. Additionally, T cells with low levels of CAR expression via RiboCAR show enhanced target cell-stimulated T cell activation, reduced markers of exhaustion and greater cytotoxicity when compared with T cells expressing CAR constitutively. CAR levels can be activated to the most effective levels and can be switched on and off according to the presence of the small molecules. With a bioavailable small molecule inducer, CAR-T activity can be precisely tuned and "remotely" controlled *in vivo*. This precise control of CAR levels with its impact on CAR-T activity and durability provides a system for significantly improving the efficacy of CAR-T therapy in comparison to current CAR-T with constitutively active CAR expression.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical stage gene therapy company with six programs in clinical development and a broad pipeline of preclinical and research programs. MeiraGTx has core capabilities in viral vector design and optimization and gene therapy manufacturing, and a transformative gene regulation platform technology that allows precise, dose-responsive control of gene expression by oral small molecules with dynamic range that can exceed 5000-fold. Led by an experienced management team, MeiraGTx has taken a portfolio approach by licensing, acquiring, and developing technologies that give depth across both product candidates and indications. MeiraGTx's initial focus is on three distinct areas of unmet medical need: ocular diseases, including both inherited retinal diseases as well as large degenerative ocular diseases, neurodegenerative diseases, and severe forms of xerostomia. Though initially focusing on the eye, central nervous system, and salivary gland, MeiraGTx plans to expand its focus to develop additional gene therapy treatments for patients suffering from a range of serious diseases.

For more information, please visit www.meiragtx.com.

Forward Looking Statement
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding our product candidate development and our pre-clinical data and reporting of such data and the timing of results of data, as well as statements that include the words "expect," "will," "intend," "plan," "believe," "project," "forecast," "estimate," "may," "could," "should," "would," "continue," "anticipate" and similar statements of a future or forward-looking nature. These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and

other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, our incurrence of significant losses; any inability to achieve or maintain profitability, raise additional capital, repay our debt obligations, identify additional and develop existing product candidates, successfully execute strategic priorities, bring product candidates to market, expansion of our manufacturing facilities and processes, successfully enroll patients in and complete clinical trials, accurately predict growth assumptions, recognize benefits of any orphan drug designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of the COVID-19 pandemic on the status, enrollment, timing and results of our clinical trials and on our business, results of operations and financial condition; failure of early data to predict eventual outcomes; failure to obtain FDA or other regulatory approval for product candidates within expected time frames or at all; the novel nature and impact of negative public opinion of gene therapy; failure to comply with ongoing regulatory obligations; contamination or shortage of raw materials or other manufacturing issues; changes in healthcare laws; risks associated with our international operations; significant competition in the pharmaceutical and biotechnology industries; dependence on third parties; risks related to intellectual property; changes in tax policy or treatment; our ability to utilize our loss and tax credit carryforwards; litigation risks; and the other important factors discussed under the caption “Risk Factors” in our most recent quarterly report on Form 10-Q or annual report on Form 10-K or subsequent 8-K reports, as filed with the Securities and Exchange Commission. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, unless required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Thus, one should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

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