Visgenx Announces Presentation of Data Supporting Potential of its Synthetic AAV DNA Platform for Gene Editing at ASGCT

Data demonstrate Synthetic AAV genome platform offers promising gene editing alternative to CRISPR based methods due to enhanced safety profile and precision

Visgenx, a retinal gene therapy Company

SAN DIEGO, CALIFORNIA, USA, May 9, 2024 /EINPresswire.com/ -- Visgenx, Inc., a pharmaceutical company focused on developing therapeutics for degenerative retinal diseases, today announced an abstract entitled "Synthetic AAV DNA Mediates High-Efficiency Homology-Directed Genome Editing" was presented at the American Society of Gene and Cell Therapy 2024 Annual Meeting being held May 7-11 in Baltimore, MD.

"We are excited to have the opportunity to present this new concept at such a prestigious conference," stated Christopher Chavez, Ph.D., of Visgenx. "We believe this data provides a promising alternative to CRISPR methods."

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> *Christopher Chavez, Ph.D.*

Unlike CRISPR, AAV vectors integrate genetic material into the host genome in a more controlled manner, reducing the risk of off-target effects and unintended mutations. However, AAV-based genome editing is inefficient, often reported below 0.1%. To overcome this limitation in efficiency, a synthetic AAV DNA system has been developed. Synthetic AAV DNA is a single-component, completely synthetic alternative to CRISPR/Cas9 or AAV-based genome editing tools that takes advantage of many of the inherent properties and versatility of AAV-based genome editing while bypassing its limitations (low editing efficiencies and manufacturing costs). In addition, synthetic AAV DNA is not bound to the payload size limitations of a standard AAV system, allowing for targeting of previously untreatable genetic diseases.

For a link to the abstract, please see: https://annualmeeting.asgct.org/abstracts/abstract-details?abstractId=60593.

ABOUT VISGENX, INC.
Visgenx, Inc. is a biotechnology company focused on developing gene-based therapeutics for degenerative retinal diseases. Visgenx' initial product is VGX-0111, a gene therapy candidate being developed for the treatment of dry Age-related Macular Degeneration (AMD). Approximately 200 million people suffer from dry AMD globally and it is a leading cause of blindness. VGX-0111 is based on the ELOVL2 gene, which is required for the biosynthesis of lipids necessary for the function and survival of retinal cells. ELOVL2 expression declines with age resulting in declining LC/VLC-PUFA levels which may be an underlying pathology of dry AMD. VGX-0111 is intended to restore a normal level of ELOVL2 expression thereby slowing or halting the vision loss resulting from dry AMD. For more information on Visgenx, visit www.visgenx.com.

Forward Looking Statements
This press release contains forward-looking statements related to Visgenx, Inc., under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995, that are subject to risks and uncertainties that could cause actual results to differ materially from those projected. Forward-looking statements include statements regarding the potential for VGX-0111 as a treatment for Dry AMD. Risks and uncertainties that could cause our actual results to differ materially and adversely from those expressed in our forward-looking statements include that the therapy may not be effective at treating Dry AMD. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release and we undertake no obligation to update any forward-looking statement in this press release except as required by law.

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