Bionano Announces Three Publications Demonstrating OGM's Utility for Cell and Gene Therapy

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- Three peer-reviewed publications collectively illustrate the building support for optical genome mapping (OGM) as a tool for cell and gene therapy development that allows researchers in academic medical centers and biopharmaceutical companies to assess genome integrity in therapeutic cell lines like chimeric antigen receptor-modified T cells (CAR-T), stem cells, and cell lines used for translational research in unraveling genetic causes of Alzheimer's disease
- Taken together, the publications illustrate that OGM can be highly sensitive for genome-wide detection of on and off target effects that may limit the utility of stem cell lines and CAR-T cell products

SAN DIEGO, May 21, 2024 (GLOBE NEWSWIRE) -- Bionano Genomics, Inc. (BNGO), today announced the publication of three studies which collectively illustrate the continued development of data supporting the utility of optical genome mapping (OGM) in cell and gene therapy applications. In all three publications, the authors note OGM's potential to provide a comprehensive landscape of genome structure and to assess genome integrity due to the workflow's ability to detect structural variants (SVs) with higher sensitivity and resolution than standard cytogenetic techniques.

Key Findings and Takeaways

In a publication from Niño Jesús University Children's Hospital (García-García *et al*.), researchers analyzed different strategies to improve the efficacy of chimeric antigen receptors (CARs) in solid tumors, focusing on the characterization of a new strategy to target neuroblastoma, based on the combination of a monoclonal antibody and fluorescein isothiocyanate (FITC) and anti-FITC CAR-T cells. Following an FDA announcement of an investigation into the serious risk of T-cell malignancy following CAR-T cell immunotherapies, the authors then evaluated the risk of genotoxicity of the CAR-T cell products, using OGM to study genomic integrity and confirm the safety of ex vivo culture and transduction of T cells. The authors noted that OGM offered a comprehensive landscape of genome structure in CAR-T cells, due to the higher sensitivity and resolution that the workflow can provide when compared to traditional methods of analysis.

- OGM analyzed PB, 45RA and CB CAR-T products after 8 days of culture and compared with T cells from donor samples at day 0 to look for new variants, including aneuploidies
- OGM revealed a single event with potential pathogenic implications in one of the analyzed donor sources, as well as additional variants of uncertain significance

The publication from Ruhr-University Bochum (Gallego Villarejo *et al*.) covered the use of OGM following gene editing to assess the genome integrity of two CRISPR/Cas9-edited hiPSC lines used for studying Alzheimer's disease. Researchers used OGM to identify on-target editing and to detect genomic alterations that might have neuroectodermal differentiation impairment. Study results confirmed the vulnerability of genomic DNA to gene editing and highlighted the utility of OGM for genome-wide quality assessment of genetic engineering.

- OGM revealed multiple aberrations that affected a large number of genes but were found to have mild impact on the ability of hiPSCs to develop cerebral organoids
- Edited hiPSCs were not found to have major phenotypic changes but one edited cell line showed potential neuroectodermal differentiation impairment
- OGM confirmed on-target edits and did not detect off-target edits

The publication from Janssen (Haidar *et al*.) details research into the Apolipoprotein E (APOE) genotype, which is the strongest risk factor for late-onset Alzheimer's disease (AD). A rare version of the APOE gene, called Christchurch, may help protect against Alzheimer's in people with a strong genetic risk for the disease. In the study, researchers describe the creation of new stem cell lines, available via the European Bank of iPSCs, where the Christchurch mutation was added to three common versions of the APOE gene to study how APOE variants impact AD and other genetic conditions. OGM was used to assess genome integrity and the data were used by researchers to support their conclusions that gene edits did not result in off-target effects.

- OGM confirmed the absence of off-target effects above 500 bp in the cell lines

"These studies highlight how researchers can use OGM when developing cell and gene therapies. The expansion of gene therapy faces risks due to both on-target and off-target structural variations that may be introduced during genome editing. Since genome aberrations caused by gene editing could obscure the true conclusions in translational research studies or even lead to unforeseen adverse effects, we believe careful and comprehensive analysis of edited genomes is important for quality control while developing these therapies and their manufacture," commented Erik Holmlin, PhD, president and chief executive officer of Bionano.

To learn more about OGM's utility in cell and gene therapy applications, please visit this website.

The publication from García-García *et al*. can be found here; the publication from Gallego Villarejo *et al.* can be found here; the publication from Haidar *et al.* can be found here.

About Bionano

Bionano is a provider of genome analysis solutions that can enable researchers and clinicians to reveal answers to challenging questions in biology and medicine. The Company's mission is to transform the way the world sees the genome through OGM solutions, diagnostic services and software. The Company offers OGM solutions for applications across basic, translational and clinical research, and nucleic acid extraction and purification solutions using proprietary isotachophoresis (ITP) technology. Through its Lineagen, Inc. d/b/a Bionano Laboratories business, the Company also provides OGM-based testing for certain laboratory developed tests. The Company also offers an industry-leading, platform-agnostic software solution, which integrates next-generation sequencing and microarray data designed to provide analysis, visualization, interpretation and reporting of copy number variants, single-nucleotide variants and absence of heterozygosity across the genome in one consolidated view. For more information, visit www.bionano.com and www.bionanolaboratories.com.

Unless specifically noted otherwise, Bionano's OGM products are for research use only and not for use in diagnostic procedures.

Forward-Looking Statements of Bionano

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "believe," "can," "may," "offer," "potential," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances and the negatives thereof) convey uncertainty of future events or outcomes and are intended to identify these forward-looking statements. Forward-looking statements include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things: the performance of OGM compared to traditional cytogenetic methods in applications for cell and gene therapy development to measure genome integrity in therapeutic cell lines including, chimeric antigen receptor-modified T cells (CAR-T), stem cells, and cell lines used for translational research in unraveling genetic causes of Alzheimer's disease; the utility of OGM in detecting on and off target events in cell and gene therapy applications described in this press release; the ability and utility of OGM to detect SVs introduced during gene editing; the ability and utility of OGM to assess genome integrity; and other statements that are not historical facts.

Each of these forward-looking statements involves risks and uncertainties. Actual results or developments may differ materially from those projected or implied in these forward-looking statements. Factors that may cause such a difference include the risks and uncertainties associated with: the impact of geopolitical and macroeconomic developments, such as the ongoing conflicts between Ukraine and Russia and Israel and Hamas, and related sanctions, global and regional pandemics, on our business and the global economy; general market conditions, including inflation and supply chain disruptions; challenges inherent in developing, manufacturing and commercializing products; our ability to further deploy new products and applications and expand the markets for our technology platforms; failure of OGM to detect SVs compared to traditional cytogenetic methods in applications for cell and gene therapy development to measure genome integrity in therapeutic cell lines including, chimeric antigen receptor-modified T cells (CAR-T), stem cells, and cell lines used for translational research in unraveling genetic causes of Alzheimer's disease; the failure of OGM to detect on and off target events in cell and gene therapy applications described in this press release; the failure of OGM to detect SVs introduced during gene editing; the failure of OGM to properly assess genome integrity; the failure of OGM to detect SVs consistent with the study results described in this press release; future study results that contradict the study results described in this press release; future study results that do not support the study results described in this press release; our expectations and beliefs regarding future growth of the business and the markets in which we operate; changes in our strategic and commercial plans; our ability to obtain sufficient financing to fund our strategic plans and commercialization efforts and to continue as a "going concern"; and including the risks and uncertainties described in our filings with the Securities and Exchange Commission, including, without limitation, our Annual Report on Form 10-K for the year ended December 31, 2023 and in other filings subsequently made by us with the Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made and are based on management's assumptions and estimates as of such date. We are under no duty to update any of these forward-looking statements after the date they are made to conform these statements to actual results or revised expectations, except as required by law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date the statements are made. Moreover, except as required by law, neither we nor any other person assumes responsibility for the accuracy and completeness of the forward-looking statements contained in this press release.

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