Study Demonstrates Potential for SMRT Sequencing to Improve the Safety of Gene Therapy Protocols

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Clinical Research Effort Establishes New Quality Control Standards and Reveals Previously Undetected Risks for Gene Therapy Delivery

MENLO PARK, Calif., March 15, 2018 (GLOBE NEWSWIRE) -- A new publication in the journal of the American Society of Gene & Cell Therapy demonstrates the utility of Single Molecule, Real-Time (SMRT®) Sequencing to assess the quality of viral vector preparations for clinical and basic research, with the ultimate goal of improving the safety and effectiveness of a type of gene therapy delivered through a viral vector. The paper was published in *Molecular Therapy — Methods & Clinical Development*

Gene therapy involves replacing a defective, disease-causing DNA sequence with a normal sequence to treat or even cure the condition. According to ClinicalTrials.gov, there are now hundreds of gene therapy-related clinical trials underway globally. Recently, scientists have begun using a harmless adeno-associated virus (AAV) as the delivery vehicle for the replacement DNA sequence; the first AAV-delivered gene therapy was approved by the U.S. Food and Drug Administration in December 2017. However, there has not been a reliable, comprehensive method for assessing the integrity of those AAV vectors prior to introducing them to patients.

This new study from scientists at the University of Massachusetts Medical School and their collaborators applied SMRT Sequencing from Pacific Biosciences of California, Inc. (Nasdaq:PACB), the leader in long-read, high-resolution sequencing, to AAV vectors, providing the first single-vector resolution view of the DNA sequences that would be delivered to a patient. Some vectors contained less than half the DNA sequence they should have, while genetic errors called chimeras were discovered in other vectors. In a patient, these vectors could have proven ineffective, reducing the likelihood of successful gene therapy outcomes. Access to this kind of information could allow gene therapy developers to make their products safer and more efficacious.

“Adeno-associated virus-based strategies have recently gained a lot of attention as an ideal vector system for delivering therapeutic transgenes in humans. We launched this study, in part, to establish quality standards that can help to define the efficacy and safety of these powerful treatments,” said Phillip Tai, co-lead author of the publication. “SMRT Sequencing allowed us to generate accurate information about vector quality that was not possible with any other technology, giving us the foundation for a method that scientists can now utilize to improve upon these promising gene therapy approaches.”

Jonas Korlach, Chief Scientific Officer of Pacific Biosciences, commented: “This is an excellent example of how the unique properties of SMRT Sequencing — including its unparalleled accuracy and single-molecule long reads — can be used to help reduce the risk of innovative medica treatments. Going forward, this approach should ultimately help make gene therapy preparation safer and more reliable.”

Publication details:

Phillip Tai et al. Adeno-Associated Virus Genome Population Sequencing Achieves Full Vector Genome Resolution and Reveals Human-Vector Chimeras. *Molecular Therapy — Methods & Clinical Development* (2018) DOI: 10.1016/j.omtm.2018.02.002

For more information, please visit http://www.pacb.com/.

About Pacific Biosciences

Pacific Biosciences of California, Inc. (NASDAQ:PACB) offers sequencing systems to help scientists resolve genetically complex problems. Based on its novel Single Molecule, Real-Time (SMRT®) technology, Pacific Biosciences’ products enable: *de novo* genome assembly to finish genomes in order to more fully identify, annotate and decipher genomic structures; full-length transcript analysis to improve annotations in reference genomes, characterize alternatively spliced isoforms in important gene families, and find novel genes; targeted sequencing to more comprehensively characterize genetic variations; and real-time kinetic information for epigenome characterization. Pacific Biosciences’ technology provides high accuracy, ultra-long reads, uniform coverage, and the ability to simultaneously detect epigenetic changes. PacBio® sequencing systems, including consumables and software, provide a simple, fast, end-to-end workflow for SMRT Sequencing. More information is available at www.pacb.com.

Forward-Looking Statements

All statements in this press release that are not historical are forward-looking statements, including, among other things, statements relating to future availability, uses, accuracy, quality or performance of, or benefits of using, products or technologies, the suitability or utility of products or technologies for particular applications or projects, and other future events. You should not place undue reliance on forward-looking statements because they involve known and unknown risks, uncertainties, changes in circumstances and other factors that are, in some cases, beyond Pacific Biosciences’ control and could cause actual results to differ materially from the information expressed or implied by forward-looking statements made in this press release. Factors that could materially affect actual results can be found in Pacific Biosciences’ most recent filings with the Securities and Exchange Commission, including Pacific Biosciences’ most recent reports on Forms 8-K, 10-K and 10-Q, and include those listed under the caption “Risk Factors.”

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