



Transplant Matches Identified by Ultra-High-Resolution HLA Typing Associated with Higher Patient Survival Rates

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In a retrospective study, Anthony Nolan Research Institute found SMRT® Sequencing identified superior HLA matches, which are critical in determining successful transplant outcomes

MENLO PARK, Calif., April 02, 2019 (GLOBE NEWSWIRE) -- Pacific Biosciences of California, Inc. (Nasdaq:PACB), the leading provider of high-quality sequencing of genomes, transcriptomes and epigenomes, today announced that a retrospective study conducted by scientists at the [Anthony Nolan Research Institute](#) in the UK demonstrated that ultra-high-resolution HLA typing performed with PacBio® sequencing identified stronger matches associated with improved survival rates among patients who received hematopoietic cell transplants. The [study](#) was published open access yesterday in the *Journal of Biology of Blood and Marrow Transplantation*.

HLA typing involves analysis of the genes found in the human leukocyte antigen region of the human genome. For stem cell transplants, HLA typing is used to find the best donor/recipient match for the strongest chance of a positive outcome for transplant patients. The HLA genes are highly polymorphic and complex, making them very difficult to resolve fully with conventional technologies. They are also known to be important in immune-related diseases and drug hypersensitivity.

The Anthony Nolan Research Institute, which is funded by [Anthony Nolan](#), a registered UK charity that maintains the world's oldest stem cell registry, has implemented Single Molecule, Real-Time (SMRT®) Sequencing from PacBio to fully phase and characterize HLA genes with high accuracy. In this retrospective study, the scientists aimed to determine whether high-resolution HLA typing enabled by SMRT Sequencing would have made a difference for previously matched donors and recipients. They analyzed 891 donor/recipient pairs, all of which had originally been considered a perfect match (a 12/12 score for all six HLA genes). SMRT Sequencing revealed that 29.1% of those matches were not actually perfect and identified previously undetected variation in nearly a quarter of the pairs. The patients whose 12/12 matches were confirmed by SMRT Sequencing had a significantly improved 5-year overall survival (OS) of 54.8% compared to 30.1% OS for those who were thought to be 12/12 matched using standard technology. Perfectly matched patients were also less likely to die of other transplant-related complications in the 12 months post-transplant, and significantly less likely to develop acute graft-versus-host disease. The study highlights the importance of sequencing through previously uncharacterized regions of the traditional HLA genes, showing that polymorphisms in these regions affect patient overall survival.

"We are very excited about this breakthrough, which has told us more than we have ever known about how to match patients and donors," said Dr. Neema Mayor, Head of Immunogenetics Research at the Anthony Nolan Research Institute. "We believe that HLA matching at ultra-high resolution could ultimately enable us to further minimize the risk of complications such as graft-versus-host disease and, consequently, the risk of mortality — potentially saving more lives in the future."

Dr. Jonas Korch, Chief Scientific Officer of Pacific Biosciences, commented: "We congratulate the Anthony Nolan research team for their commitment to continually pushing forward to develop better methods of investigating molecular differences that can influence the outcomes of stem cell transplantation. These results offer additional evidence that using SMRT Sequencing to characterize polymorphisms in HLA genes outside of the protein binding regions is critical for strengthening causality association studies."

For more information, please visit <https://www.pacb.com/applications/targeted-sequencing/hla/>.

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.

About Anthony Nolan

Anthony Nolan saves the lives of people with blood cancer. The charity uses its register to match potential stem cell donors to blood cancer and blood disorder patients in need of stem cell transplants. It also carries out pioneering research to increase stem cell transplant success and supports patients through their transplant journeys. Since 1974, Anthony Nolan has been at the cutting edge of stem cell transplantation. In 2016, it launched new third-generation sequencing (TGS) in its SMARTLAB® – a revolution in lifesaving technology, and the most significant breakthrough in matching donors with transplant recipients since the introduction of peripheral blood stem cell donation (PBSC) in the year 2000. For more information, visit <https://www.anthonynolan.org/>.

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 methods, products or technologies for particular applications, studies or projects, the expected benefits of this study or of HLA typing using SMRT Sequencing, 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."

Pacific Biosciences undertakes no obligation to revise or update information in this press release to reflect events or circumstances in the future, even if new information becomes available.

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