GenieUs Genomics, Duke and Temple Universities - Tackle ALS Heterogeneity with Genomic Profiling in New Clinical Trial

Deep Sequencing from PacBio used by GenieUs Genomics, Duke University and Temple University for a Precision Health Approach in ALS Clinical Trial

SYDNEY, NSW, AUSTRALIA, June 26, 2024 /EINPresswire.com/ -- GenieUs Genomics (GenieUs), in collaboration with Duke University and Temple University, is launching the first-ever precision health approach to patient stratification in a clinical research study for amyotrophic lateral sclerosis (ALS) in the United States. This initiative, titled "ROAR-DiGAP: A Widely Inclusive, Largely Virtual Pilot Trial Utilizing DiGAP™ (Deep Integrated Genomics Analysis Platform) To Personalize Treatments", marks a significant advancement in the quest to understand and treat ALS through personalized medicine.

Led by researchers and neurologists from Duke University, Temple University, and GenieUs, this innovative approach will assess precision medicine for ALS. At the forefront of this endeavor is DiGAP™, a bioinformatic platform developed by GenieUs. DiGAP™ will provide comprehensive genomic profiling and stratify 50 enrolled participants living with ALS into four categories based on their Pathway Mutation Burden - neuroinflammation, oxidative stress, impaired autophagy & axonal transport, and mitochondrial dysfunction. Each category will receive an individualized treatment targeting the pathway category, and effects on the Revised ALS Functional Rating Scale (ALSFRS-R) progression, a panel of mechanistic biomarkers and neurofilament light chain measurements, will be measured over the 9-month study.

DiGAP™ strives to uncover genetic variants and signatures of ALS subtypes, which may serve as digital genomic biomarkers of molecular drivers of ALS in individual patients. This study also aims to assess genomic signatures that distinguish therapy responders from non-responders.

To accomplish those goals, the team will utilize the extraordinary power of the Pacific Biosciences (PacBio) Revio system and HiFi long-read sequencing. HiFi reads make it possible to find genomic signatures that other technologies struggle to uncover. This includes critical information such as phasing (e.g., which of the two chromosomes a mutation occurred on), DNA methylation status, and the detection of insertions, deletions, and structural variants.

Dr. Sherie Ma, GenieUs Genomics' CEO said, "Our technology seeks to uncover ALS subtypes, we believe genomic profiling is the key to developing effective therapies for diseases with a high level of heterogeneity, such as ALS. This is an excellent application of DiGAP's™ genomic profiling capabilities which aims to improve the chances of success in clinical trials."

Dr. Richard S. Bedlack MD, PhD, Stewart, Hughes and Wendt Distinguished Professor and Director of ALS Program at Duke University School of Medicine, commented: "All the compounds tested in this program are associated with at least one case of recovery from ALS (ALS reversal) and all the studies are widely inclusive, largely virtual, and use historical (not placebo) controls. Here, for the first time, we will try to match each participant's biology to a different specific compound for a 'personalized medicine' approach."

Dr. Terry Heiman-Patterson, MD, Professor of Neurology and Director of the MDA/ALS Center of Hope at the Lewis Katz School of Medicine at Temple University added, "I feel privileged to be working with Dr. Bedlack to finally approach ALS treatment by stratifying people based on disease biology. This is in contrast to previous attempts where the heterogeneous ALS population has been lumped together and treated the same, despite understanding that this is not a single disease. This has likely resulted in discarding treatments that would work in subgroups of people living with ALS."

Jeff Eidel, Chief Commercial Officer at PacBio added that "PacBio feels truly honoured to be a part of this study, reaffirming our commitment to advancing the understanding and possible treatment of ALS and other neurodegenerative diseases. With PacBio technology, we're enabling the possibility of deeper insights and more complete genetic characterization that only long-read sequencing can provide."

For more information about the clinical trial: https://clinicaltrials.gov/study/NCT06429059.

About GenieUs Genomics
GenieUs Genomics is an early-stage bioinformatics company based in Sydney, Australia. Our mission is to accelerate precision therapies for ALS by decoding the genetics of neurodegeneration. Our core technology, the Deep integrated Genomic Analysis Platform - DiGAP™, is a fully automated, end-to-end platform for high-throughput and robust whole patient genome decoding and analysis. We harness AI and ML data to enhance clinical outcomes through genome-guided patient stratification and facilitate drug discovery, to advance development of better therapeutics for neurodegenerative diseases.

About Duke University ALS Program
The Duke University ALS Program consists of one of the world's largest, most comprehensive multi-disciplinary clinics (www.dukealsclinic.org). Innovative patient education initiatives include the ALS Clinical Research Learning Institute which empowers people living with ALS to be even more effective research advocates (https://pubmed.ncbi.nlm.nih.gov/32458763/). Patient-centric

ALS research includes ALSUntangled (www.alsuntangled.org) which helps patients make informed decisions about alternative and off-label treatments, and ALS Reversals to discover why some people with ALS recover from it.

About Lewis Katz School of Medicine at Temple University MDA/ALS Center of Hope
The MDA/ALS Center of Hope at the Lewis Katz School of Medicine at Temple University is a multidisciplinary ALS clinic serving the greater Philadelphia Region since 2016. The center is dedicated to excellence in clinical care of people living with ALS with a patient centric approach. The center also actively participates in clinical research including trials, tissue banking, natural history studies, and research leveraging technologies.

Forward-Looking Statements
This press release may contain "forward-looking statements" within the meaning of Section 21E of the Securities Exchange Act of 1934, including statements relating to advancing the understanding and possible treatment of ALS and other neurodegenerative diseases, and the use of PacBio technology in the study for deeper insights and more complete genetic characterization, among other statements. You should not place undue reliance on forward-looking statements because they are subject to assumptions, risks and uncertainties, which could cause actual outcomes and results to differ materially from currently anticipated results. Factors that could materially affect actual results can be found in PacBio's most recent filings with the Securities and Exchange Commission, including PacBio's most recent reports on Forms 8-K, 10-K, and 10-Q, and include those listed under the caption "Risk Factors." These forward-looking statements are based on current expectations and speak only as of the date hereof; except as required by law, PacBio disclaims any obligation to revise or update these forward-looking statements to reflect events or circumstances in the future, even if new information becomes available.

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