GenieUs Genomics Announces Completion of Phase 2 Clinical Trial Enrollment

A Key Step Forward in Addressing the Heterogeneity of ALS to Unlock Precision Treatments

SYDNEY, NSW, AUSTRALIA, October 2, 2024 /EINPresswire.com/ -- GenieUs Genomics (GenieUs), in collaboration with trial sites at Duke University School of Medicine and the Lewis Katz School of Medicine at Temple University, today announces the completion of enrollment of 50 patients in the Phase 2 clinical trial, ROAR-DiGAP, focused on precision health and patient stratification in amyotrophic lateral sclerosis (ALS). The trial is aimed at advancing the use of personalized medicine in ALS research.

At the forefront of this endeavor is DiGAP™, a bioinformatic platform developed by GenieUs. DiGAP™ is designed to provide comprehensive genomic profiling and to stratify participants into four categories based on their Pathway Mutation Burden - neuroinflammation, oxidative stress, impaired autophagy & axonal transport, and mitochondrial dysfunction. Each category will be treated with an individualized treatment tailored to 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 nine-month study.

DiGAP™ strives to uncover genetic variants and signatures of ALS subtypes, which may potentially serve as digital genomic biomarkers of molecular drivers of ALS in individual patients. This study also aims to assess genomic biomarkers that distinguish therapy responders from non-responders. An advanced genomic sequencing technology has been adopted for the study -- the PacBio Revio system and HiFi long-read sequencing -- to uncover genomic signatures potentially missed by other technologies.

Enrollment began in June 2024, and the rapid recruitment reflects the study's inclusive and primarily virtual design, allowing for simpler participation and broader accessibility.

Dr. Sherie Ma, GenieUs Genomics' CEO said, " A heartfelt thank you to the patients and families involved in our clinical program. The rapid enrollment reflects the significant unmet need for more effective, targeted therapies for ALS. We are making meaningful progress toward developing personalized treatments that could improve the lives of those living with ALS, and we

look forward to receiving trial results by the second quarter of 2025. Through genomic profiling, DiGAP aims to further address the heterogeneity across ALS patients and contribute to further our understanding of the etiology of the disease."

Dr. Richard S. Bedlack MD, PhD, Stewart, Hughes and Wendt Distinguished Professor and Director of ALS Program at Duke University School of Medicine, commented: "I am thrilled to combine the patient-centric features of my ROAR trials with the personalized medicine approach. The fast enrollment confirms that our patient-partners share in this excitement."

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, "The personalized and patient centric approach that is reflected in the ROAR-DiGAP trial could form a model for future progress in ALS treatment development. It will ensure focused treatments and underscores the importance of patient stakeholder inclusion."

Jeff Eidel, Chief Commercial Officer at PacBio commented "We are thrilled to support this groundbreaking study and are excited to see the insights that will emerge from using PacBio's HiFi sequencing technology. The precision and comprehensive data our platform provides have proven invaluable in uncovering genetic signatures that can lead to more targeted and effective treatments for ALS."

About GenieUs Genomics
GenieUs Genomics' is bioinformatics company based in Sydney, Australia. Our mission is to accelerate precision therapies for Amyotrophic Lateral Sclerosis (ALS) by decoding the genetics of neurodegeneration. Our core technology, the Deep integrated Genomic Analysis Platform - DiGAP™, is a fully automated platform for high-throughput whole patient genome decoding and analysis. We discover data-driven insights to enhance clinical outcomes through genome-guided patient stratification, facilitating the development of novel therapeutics in a disease characterised by a high level of heterogeneity.

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 more informed decisions about alternative and off-label treatments, and ALS Reversals which attempts to discover why some people with ALS recover from it, and to make this happen more often.

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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