PacBio Announces Plans for Collaboration With n-Lorem Foundation and EspeRare to Advance Precision Therapies for Rare Genetic Diseases

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The planned collaboration highlights the role of complete genome resolution in scaling n-of-1 therapeutic models

MENLO PARK, Calif., Jan. 12, 2026 (GLOBE NEWSWIRE) -- PacBio (NASDAQ: PACB), developer of the world's most advanced sequencing technologies, today announced plans to pursue a strategic collaboration with the n-Lorem Foundation and EspeRare that we believe will position long-read whole-genome sequencing as enabling infrastructure to support the development of individualized antisense oligonucleotide (ASO) therapies for ultra-rare genetic diseases.

Under the proposed collaboration, PacBio will provide its scientific expertise, and the collaboration will utilize PacBio's long-read whole genome sequencing technology, to study the design, feasibility assessment, and molecular characterization of ASO therapies – to help extend the role of long-read genomics for those who may lack effective treatment options.

ASO therapies require a complete understanding of the genomic architecture, including variant phasing, structural variation, repeat expansions, and regulatory alterations that are frequently missed or unresolved by standard sequencing approaches. By integrating long-read whole-genome sequencing earlier in the therapeutic development process, the proposed collaboration will aim to reduce the need for multiple sequential tests, accelerate target validation, and improve confidence in ASO design.

Under the proposed collaboration, PacBio will donate sequencing reagents and provide expert scientific resources to support long-read genome analysis for research to further development of therapeutic treatments for individuals with rare diseases. These efforts are intended to directly inform target identification, ASO design feasibility, and regulatory-grade molecular characterization, strengthening the end-to-end pipeline from genomic discovery to enabling development of therapeutic interventions.

"Rare disease therapies demand a level of genomic resolution that simply isn't achievable with fragmented testing approaches," said Christian Henry. "By working with n-Lorem and EspeRare under the proposed collaboration, we aim to establish HiFi long-read whole genome sequencing as the preferred platform for precision therapeutic development in rare disease, where understanding the full complexity of the genome is essential to designing safe and effective interventions."

n-Lorem Foundation is a nonprofit organization dedicated to discovering, developing, and providing free, individualized ASO therapies for patients with rare genetic diseases, often under patient-specific regulatory pathways. The EspeRare foundation works to unlock and advance translational infrastructure, partnerships, and patient access to novel therapeutics across rare disease communities. Together, the organizations represent a complementary ecosystem spanning patient identification, molecular characterization, therapy development, and clinical implementation.

"n-Lorem was founded to discover, develop, manufacture and provide experimental ASO treatments to patients with the rarest genetic mutations for free, for life. To bring genetic medicines to patients with extremely rare genetic diseases, the first step is to understand the genomic architecture of each patient," said Stanley T. Crooke, M.D., Ph.D., Founder, chairman and CEO, n-Lorem Foundation. "PacBio's generous donation of long-read sequencing for many patients each year will enhance our ability to meet the extraordinary demand from the nano-rare community. It exemplifies the heart of the therapeutic industry, and it adds to the generosity of so many companies in our industry that have stepped up to help n-Lorem do what seemed impossible six years ago."

"PacBio's long-read sequencing technology enables the level of genomic understanding required to guide care in rare diseases," said Caroline Kant, Executive Director of EspeRare. "Through the proposed collaboration we will be able to translate complex genomic information into therapies tailored to each patient's biology, bringing groundbreaking treatments to families who urgently need them."

As individualized and n-of-1 therapeutic models gain momentum across the biopharma industry, PacBio aims to play a central role in supporting scalable, genomics-driven therapy development, where complete and accurate genome characterization is foundational to developing treatment options.

The proposed collaboration reflects PacBio's broader commitment to advancing human health through technologies that enable deeper biological insight and support the development of potential actionable outcomes.

About PacBio
PacBio (NASDAQ: PACB) is a premier life science technology company that designs, develops, and manufactures advanced sequencing solutions to help scientists and clinical researchers resolve genetically complex problems. Our products and technologies, which include our HiFi long-read sequencing, address solutions across a broad set of research applications including human germline sequencing, plant and animal sciences, infectious disease and microbiology, oncology, and other emerging applications. For more information, please visit www.pacb.com and follow @PacBio.

PacBio products are provided for Research Use Only. Not for use in diagnostic procedures.

About n-Lorem Foundation
n-Lorem Foundation is a non-profit organization established to apply the efficiency, versatility and specificity of antisense technology to charitably provide experimental antisense oligonucleotide (ASO) medicines to treat nano-rare patients diagnosed with diseases that are the result of a single genetic defect unique to only one or very few individuals. Nano-rare patients describe a very small group of patients (1-30 worldwide) who, because of their small numbers, have few if any treatment options. n-Lorem Foundation was created to provide hope to these nano-rare patients by developing individualized ASO medicines, which are short strands of modified DNA that can specifically target the transcripts of a defective gene to correct the abnormality. The advantage of experimental ASO medicines is that they can be developed rapidly, inexpensively and are highly specific. To date,

n-Lorem received over 380 applications for treatment with more than 200 nano-rare patients approved and more than 30 patients on treatment. n-Lorem was founded by Stanley T. Crooke, M.D., Ph.D., former chairman and CEO of Ionis Pharmaceuticals, who founded Ionis Pharmaceuticals in 1989 and, through his vision and leadership, established the company as the leader in RNA-targeted therapeutics. Follow us on Twitter, Facebook, LinkedIn and YouTube.

To learn more about n-Lorem's mission visit us at www.nlorem.org, and please consider giving to n-Lorem to bring hope, possibility and treatment options to nano-rare patients in need.

About EspeRare
EspeRare is a nonprofit organization dedicated to advancing therapies for patients with rare diseases through strategic partnerships, translational infrastructure, and patient-centered programs. After a decade of removing drug development barriers that prevent promising science from reaching historically overlooked patients, EspeRare has become a pioneer in prenatal and personalized therapies for rare diseases. Combining biotech agility with a mission-driven model, EspeRare bridges the translational "valley of death" by aligning patient communities, academics, commercial partners, and regulators to advance treatments and support equitable access. Based in Geneva, it leverages its position at the crossroads of science, health diplomacy and the life science industry to structure collaborations that unlock innovative therapies and maximize global patient impact.

Forward Looking Statements
This press release may contain "forward-looking statements" within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and the U.S. Private Securities Litigation Reform Act of 1995. All statements other than statements of historical fact are forward-looking statements, including statements relating to: the uses, advantages, or quality or performance of, or benefits or expected benefits of using, PacBio products or technologies, including in connection with the proposed collaboration; entering into the proposed collaboration; outcomes, results, developments and impacts of, and the positioning of PacBio products in connection with or as a result of, the proposed collaboration, such as reducing the need for multiple tests, acceleration of target validation, and improved confidence in therapeutic development and design, resulting from the use of PacBio products; use of PacBio products to translate genomic information into individually-tailored; and other future events. You should not place undue reliance on forward-looking statements because they are subject to assumptions, risks, and uncertainties and could cause actual outcomes and results to differ materially from currently anticipated results, including, challenges in negotiating and entering into collaboration arrangements with multiple parties and risk that PacBio may be unable to agree to terms on the proposed collaboration; challenges inherent in generating, validating and accelerating discoveries in connection with, and supporting the potential development of, individually-tailored genomics-related therapies; potential product performance and quality issues; regulatory issues related to therapeutic development; and third-party claims alleging infringement of patents and proprietary rights or seeking to invalidate PacBio's patents or proprietary rights, among others. Additional 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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