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NeuBase Therapeutics Reports Business Update and Financial Results for Fiscal Year 2022

PITTSBURGH, Dec. 21, 2022 (GLOBE NEWSWIRE) -- NeuBase Therapeutics, Inc. (Nasdaq: NBSE) ("NeuBase" or the "Company"), a biotechnology platform company Drugging the Genome™ to address disease at the base level using a new class of precision genetic medicines, today reported its financial results for the fiscal year ended September 30, 2022, and other recent developments.

"Fiscal year 2022 was a challenging period in our Company's history as we faced a tightening of the broader capital markets for small biotech companies. To address this reality, we made the decision to reduce internal investments in our gene silencing programs in myotonic dystrophy type 1 (DM1), Huntington's disease (HD) and cancers driven by common *KRAS* gene mutations, which were entering more expensive development stages. In order to keep building momentum for these programs as they move into the clinic and beyond, we are pursuing collaborative initiatives, including partnerships. We believe potential collaborators will be attracted by the important new exploratory toxicology data in non-human primates (NHP) and rodents we generated in our DM1 program, and new three-month NHP pharmacokinetic data, illustrating that we can deliver pharmacologically effective amounts of our development candidate to tissues affected in DM1 patients with doses under the maximum tolerated doses, possibly unlocking a path to an Investigational New Drug (IND) filing and human clinical trials," stated Dietrich A. Stephan, Ph.D., Founder and Chief Executive Officer of NeuBase.

"Our technology platform is unique in that it allows us to engage the double stranded genome in a sequence selective manner to modulate machinery that controls gene function. We have now illustrated the capability to silence disease-gene output in various high-value indications. As mentioned, we are focused on moving these programs forward via partnerships. Our technology has also been repeatedly published in journals such as *Nature* and *Science* to perform 'nuclease free' *in vivo* gene editing to restore healthy gene function. In the future, we expect to develop the capability to increase gene output to address loss-of-function mutations. It is this ability to address the three major causal mechanisms behind genetic diseases that promises a unified and scalable approach to addressing disease in the future. FY2022 is a turning point as we take the next step in the expansion of the capabilities of the platform and expect to generate an exciting next generation of therapies capable of addressing various high-value genetic mutations."

"The excitement around our gene editing technology is driven by our ability to achieve precise repair of a diverse set of mutations in the genome without the need to deliver nucleases, such as modified bacterial CRISPR/Cas enzymes. To achieve this, we create platform-derived compounds that target a mutation, without the requirement for a flanking protospacer adjacent motif (PAM) sequence, and recruit the cell's own DNA repair

machinery to correct transition, transversion, insertion and deletion mutations, thus potentially enabling correction of up to 90% of all known pathogenic mutations. The system utilizes four layers of sequence selectivity to dramatically increase fidelity and reduce or eliminate off-target edits. A low immunogenicity profile alongside low toxicity due to a lack of double-stranded breaks enables the potential for repeat dosing to compensate for tissue turnover and achieve requisite editing efficiencies needed for clinical benefit. We plan to use non-viral delivery technologies to further bolster the ability to repeatedly dose without generating an acquired immune response, to ensure durable disease management. This editing approach leverages machinery that is used up to a million times a day, in each of the trillions of cells in the human body, to ensure the fidelity of our hereditary information, speaking to why the fidelity metrics that have been published using our technology significantly outperform the base and prime editors, and position the system favorably for *in vivo* applications. Our PATrOL™ editing technology recently attracted a global healthcare company to collaborate with us in a research agreement to evaluate three monogenic genetic diseases."

"As we look ahead to fiscal year 2023, we expect to announce partnerships that move our gene silencing programs forward while ensuring that learnings and validating data are available to the Company so we can continue to improve our platform performance over time. We also expect to announce an expanded pipeline that includes gene editing programs targeting prevalent mutations that cannot be edited by base editors. Throughout calendar year 2023, we anticipate sharing data on *ex vivo* and *in vivo* editing results against high-value genetic mutations, together with associated performance metrics such as fidelity and efficiency," concluded Dr. Stephan.

Fourth Quarter of Fiscal Year 2022 and Recent Operating Highlights

- **R&D Expansion to Include Gene Editing:** The Company announced plans to expand its R&D focus to include the advancement of the differentiated gene editing capabilities of the Company's PATrOL™ platform; currently identifying and evaluating multiple indications for possible future development; further details expected to be provided during fiscal year 2023 regarding the Company's gene editing pipeline.
- **Research Agreement:** In line with the expansion of NeuBase's focus to include the advancement of its platform into gene editing, the Company announced a research agreement with a global healthcare company, pursuant to which the global healthcare company will evaluate the PATrOL™ platform for three monogenic genetic diseases and collaborate with NeuBase on the evaluation of drug candidates for three undisclosed indications. The global healthcare company will have the exclusive opportunity, subject to certain terms and conditions, to license and develop the drug candidates created under this research evaluation agreement.
- **Gene Silencing Pipeline Strategy:** The Company is actively pursuing collaborative initiatives, including partnerships, for its DM1, HD, and KRAS (G12D and G12V) programs. Preclinical activities for all three of these programs have been deferred, and plans to submit an IND application for DM1 to the U.S. Food and Drug Administration (FDA) are on hold.
- **Corporate Restructuring:** The Company completed a workforce reduction of approximately 60% and implemented other cost reduction plans to extend its cash runway into the second quarter of calendar year 2024 based on current operating plans and estimates.

- **Board of Directors Update:** Dov A. Goldstein, M.D. was appointed Chairperson of the Company's Board of Directors effective October 14, 2022.

Financial Results for the Fiscal Year Ended September 30, 2022

- As of September 30, 2022, the Company had cash and cash equivalents of approximately $23.2 million, compared with approximately $52.9 million as of September 30, 2021.
- NeuBase estimates its current cash and cash equivalents are sufficient to fund currently planned operating and capital expenditures into the second quarter of calendar year 2024.
- For the fiscal year ended September 30, 2022, the Company reported a net loss of approximately $33.8 million, or a net loss of $1.04 per share, compared with a net loss of approximately $25.4 million, or a net loss of $0.93 per share, for the same period last year.
- For the fiscal year ended September 30, 2022, total operating expenses were approximately $33.3 million, consisting of approximately $11.9 million in general and administrative expenses and $21.4 million of research and development expenses. This compares with total operating expenses of approximately $26.6 million for the same period last year, consisting of approximately $12.2 million in general and administrative expenses, $11.5 million in research and development expenses and $2.9 million in research and development expenses related to the acquisition of assets of Vera Therapeutics, Inc.

About NeuBase Therapeutics
NeuBase is accelerating the genetic revolution by developing a new class of precision genetic medicines that Drug the Genome™. The Company's therapies are built on a proprietary platform called PATrOL™ that encompasses a novel peptide-nucleic acid antisense oligonucleobase technology combined with a novel delivery shuttle that overcomes many of the hurdles to selective mutation engagement, repeat dosing, and systemic delivery of genetic medicines. To learn more, visit www.neubasetherapeutics.com.

Use of Forward-Looking Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act. These forward-looking statements are distinguished by the use of words such as "will," "would," "anticipate," "expect," "believe," "designed," "plan," or "intend," the negative of these terms, and similar references to future periods. These forward-looking statements include, among others, those related to the potential and prospects of the Company's proprietary PATrOL™ platform. These views involve risks and uncertainties that are difficult to predict and, accordingly, our actual results may differ materially from the results discussed in our forward- looking statements. Our forward-looking statements contained herein speak only as of the date of this press release. Factors or events that we cannot predict, including those risk factors contained in our filings with the U.S. Securities and Exchange Commission (the "SEC"), may cause our actual results to differ from those expressed in forward-looking statements. The Company may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in the forward-looking statements, and you should not place undue reliance on these forward-looking statements. Because such statements deal with future events and are based on the Company's current expectations, they are subject to various risks and

uncertainties, and actual results, performance or achievements of the Company could differ materially from those described in or implied by the statements in this press release, including: the Company's plans to research, develop and commercialize any product candidates; the timing of initiation of any clinical trials; the risk that prior data will not be replicated in future studies; the timing of any investigational new drug application or new drug application; the clinical utility, potential benefits and market acceptance of any product candidates; the Company's commercialization, marketing and manufacturing capabilities and strategy; global health conditions, including the impact of COVID-19; the Company's ability to protect its intellectual property position; and the requirement for additional capital to continue to advance these product candidates, which may not be available on favorable terms or at all, as well as those risk factors contained in our filings with the SEC. Except as otherwise required by law, the Company disclaims any intention or obligation to update or revise any forward-looking statements, which speak only as of the date hereof, whether as a result of new information, future events or circumstances or otherwise.

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