May 11, 2023

NeuBase Reports Business Update and Financial Results for the First Quarter of 2023

Announced oral presentations at the American Society of Gene & Cell Therapy ("ASGCT") 26th Annual Meeting to take place on May 16-20, 2023

Company to present additional data showcasing gene editing capabilities at scientific conferences throughout remainder of 2023

PITTSBURGH, May 11, 2023 (GLOBE NEWSWIRE) -- NeuBase Therapeutics, Inc. (Nasdaq: NBSE) ("NeuBase" or the "Company"), a biotechnology company developing Stealth Editors™ to perform *in vivo* gene editing without triggering the immune system, today reported its financial results for the three-month period ended March 31, 2023 and other recent developments.

"To date in 2023, we have made steady progress advancing the development of our gene editing application based on our PATrOL™ platform. One of the main drivers behind the initial success of this application area, and speed at which we are moving forward, is the ability to leverage our extensive experience in peptide nucleic acids ("PNAs"), the chemistry which forms the core of our platform. PNAs have been shown to engage the double-stranded human genome due to their high binding affinity and exquisite sequence selectivity. This chemistry would allow us to tag a locus in the genome that harbors a mutation with a simple synthetic compound and recruit the cell's own machinery to repair the mutation. This is the approach behind our *in vivo* gene editing platform," stated Dietrich A. Stephan, Ph.D., Founder and Chief Executive Officer of NeuBase. "We believe our Stealth Editors™ can potentially address up to 90% of disease-causing mutations, compared to the estimated approximately 20% addressed by base editing platforms currently in development. In addition, our approach to gene editing offers several benefits that differentiate us from CRISPR/Cas editors, base editors, and prime editors. For example, Stealth Editors™ are non-immunogenic and have exquisite fidelity, maximizing the ability to re-dose should durability wane over time, which is a situation we have seen with gene therapy approaches in the past."

"Throughout the remainder of the year we look forward to presenting initial *ex vivo* and *in vivo* editing results against high-value genetic mutations, together with associated performance metrics, such as fidelity and efficiency. This includes preclinical data in two oral presentations the NeuBase team will provide at the ASGCT 26th Annual Meeting taking place next week. We plan to follow up the presentations with an investor and analyst conference call on May 22, 2023 to discuss these data," concluded Dr. Stephan.

First Quarter of 2023 and Recent Operating Highlights

- **Gene Editing Program:**
  - The Company is advancing development of the differentiated gene editing capabilities of its PATrOL™ platform and development of Stealth Editors™.
  - Two abstracts accepted for oral presentations at the ASGCT 26th Annual Meeting, which is being held in Los Angeles, CA and virtually on May 16-20, 2023. Management plans to hold an investor and analyst conference call on May 22, 2023.
  - Details of the gene editing pipeline expected to be provided during 2023.
- **Gene Editing Research Agreements:**
  - The Company's previously announced collaboration with a global healthcare company to evaluate the PATrOL™ platform for three monogenic genetic diseases remains on track. 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.
  - Engaged in discussions with other healthcare companies on the potential for additional research agreements.
- **Gene Silencing Pipeline Collaborations:**
  - NeuBase continued active pursuit of collaborative initiatives, including partnerships, for the Company's myotonic dystrophy type 1, Huntington's disease, and KRAS (G12D and G12V) programs. The Company currently expects that any collaborations that could result from these discussions would be announced in the second half of 2023.

Financial Results for the Quarter Ended March 31, 2023

- As of March 31, 2023, the Company had cash and cash equivalents of approximately $13.8 million, compared with approximately $17.4 million as of December 31, 2022.
- NeuBase estimates its current cash and cash equivalents are sufficient to fund currently planned operating and capital expenditures into the second quarter of 2024.
- For the quarter ended March 31, 2023, the Company reported a net loss of approximately $4.1 million, or a net loss of $0.12 per share, compared with a net loss of approximately $9.9 million, or a net loss of $0.30 per share, for the same period last year.
- For the quarter ended March 31, 2023, total operating expenses were approximately $4.2 million, consisting of approximately $2.9 million in general and administrative expenses and $1.3 million in research and development expenses. This compares with total operating expenses of approximately $9.9 million for the same period last year, consisting of approximately $3.1 million in general and administrative expenses and $6.8 million in research and development expenses.
- On April 21, 2023, the Company's Board of Directors approved a change in its fiscal year from the twelve months beginning October 1 and ending September 30 to the twelve months beginning January 1 and ending December 31, which will be effective for the year ending December 31, 2023.

About NeuBase Therapeutics

NeuBase is a pre-clinical stage biopharmaceutical company leveraging its peptide-nucleic acid technology to accelerate the genome editing revolution. NeuBase's Stealth Editing™ technology is a new type of gene editing designed to avoid being identified by the immune system and provide pronounced effects that are safe, delivered with non-viral technologies,

and broadly applicable across different mutation types and industries. This *in vivo* gene editing system seeks to address disease at the base level by recruiting the body's own editing machinery to correct mutations that cause disease. The Company projects that its technology can potentially address up to ~90% of all known human mutations, including insertions, deletions, transitions, and transversions with a simple non-immunogenic solution. 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," "project," 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 or Stealth Editing™ technology, the Company's plans to announce details regarding its internal programs, statements regarding potential collaborations and the Company's estimate that its current cash and cash equivalents are sufficient to fund currently planned operating and capital expenditures into the second quarter of 2024. 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 cash expenditures; 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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