UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K
Current Report
Pursuant to Section 13 or 15(d) of the
Securities Exchange Act of 1934

Date of report (Date of earliest event reported): May 22, 2023

NeuBase Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	001-35963	46-5622433
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

350 Technology Drive, Pittsburgh, PA	15219
(Address of Principal Executive Offices)	(Zip Code)

(412) 763-3350
(Registrant's Telephone Number, Including Area Code)

N/A
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	NBSE	The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR § 230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR § 240.12b-2).
Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01. Regulation FD Disclosure.

Announcement of Preclinical Data

On May 22, 2023, NeuBase Therapeutics, Inc. (the "Company") conducted a webcast and conference call with investors to discus a broad set of preclinical safety and efficiency data for its Stealth Editors™ development program. The presentation materials for the webcast and conference call and the Company's press release related to these data are furnished herewith as Exhibits 99.1 and 99.2, respectively, to this Current Report on Form 8-K (this "Current Report"). The webcast presentation will be archived and accessible at https://ir.neubasetherapeutics.com/news-events/ir-calendar.

The information contained in this Item 7.01 of this Current Report, including Exhibits 99.1 and 99.2 hereto, is being furnished pursuant to Item 7.01 and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, and it shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or under the Exchange Act, whether made before or after the date hereof, except as expressly set forth by specific reference in such filing to this Item 7.01 of this Current Report.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
99.1	Preclinical Data Presentation Materials for webcast and conference call held on May 22, 2023
99.2	Press Release, dated May 22, 2023
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

NEUBASE THERAPEUTICS, INC. (Registrant)

Date: May 22, 2023

By: /s/ *Todd P. Branning*
Todd P. Branning
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 99.1

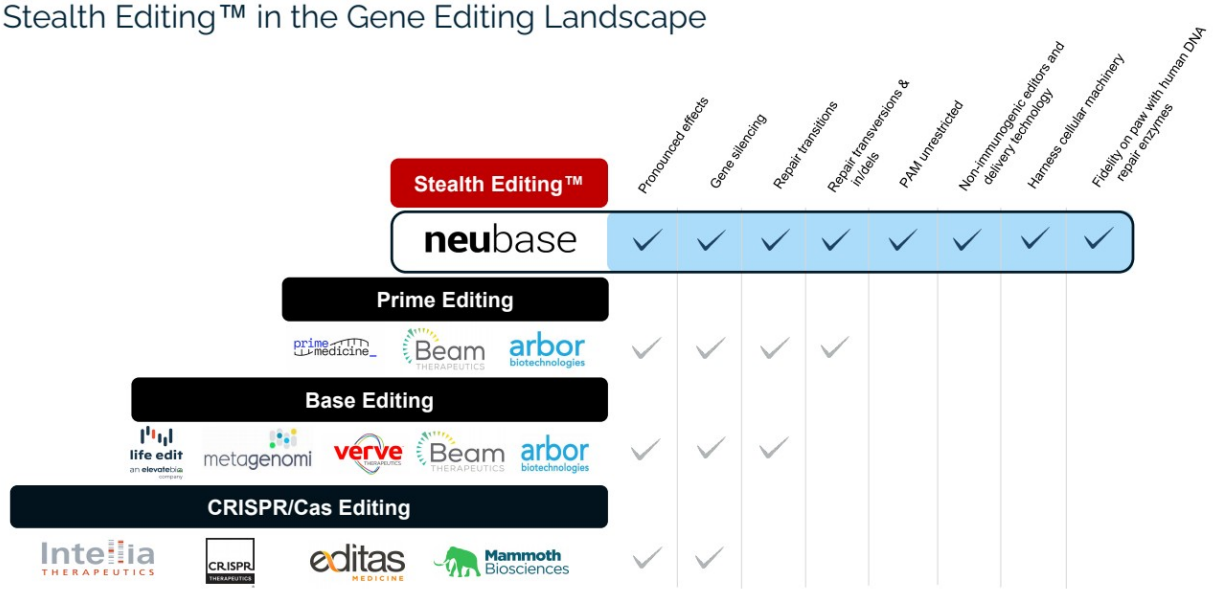

neubase
The Future of Gene Editing is Stealth Editing™
Investor Conference Call | May 22, 2023

Safe Harbor Statement

Certain statements contained in this presentation regarding matters that are not historical facts are forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and the Private Securities Litigation Reform Act of 1995, known as the PSLRA. These include statements regarding management's intentions, plans, beliefs, expectations or forecasts for the future, and, therefore, you are cautioned not to place undue reliance on them. No forward-looking statement can be guaranteed, and actual results may differ materially from those projected.

NeuBase Therapeutics, Inc. ("NeuBase") undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise, except to the extent required by law. NeuBase uses words such as "anticipates," "believes," "plans," "expects," "projects," "future ," "intends," "may," "will," "should," "could," "estimates," "predicts," "potential," "continue," "guidance," the negative of these terms, and similar expressions to identify these forward-looking statements that are intended to be covered by the safe harbor provisions of the PSLRA. Such forward-looking statements are based on NeuBase's expectations and involve risks and uncertainties; consequently, actual results may differ materially from those expressed or implied in the statements due to a number of factors, including, but not limited to, NeuBase'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; NeuBase's commercialization, marketing and manufacturing capabilities and strategy; global health conditions, including the impact of COVID-19; NeuBase's ability to protect its intellectual property position; and the requirement for additional capital to continue to advance NeuBase's product candidates, which may not be available on favorable terms or at all. New factors emerge from time to time and it is not possible for NeuBase to predict all such factors, nor can NeuBase assess the impact of each such factor on the business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. Forward-looking statements included in this presentation are based on information available to NeuBase as of the date of this presentation. NeuBase disclaims any obligation to update such forward-looking statements to reflect events or circumstances after the date of this presentation, except as required by applicable law. This presentation does not constitute an offer to sell, or the solicitation of an offer to buy, any securities.

Stealth Editing™ in the Gene Editing Landscape



Immunogenicity from viral delivery systems and bacterial proteins presents a potential safety issue for patients receiving gene editing therapies

Stealth Editors™ - designed to fly under the radar of the immune system to effect high fidelity gene editing

Non-viral Delivery

With a non-immunogenic system that can reach diverse tissues and cell types via systemic administration

Harnesses Cellular Mechanisms

Through the utilization of DNA repair machinery that has been refined over millennia to deliver precise on-target edits with minimal off-target edits

Pronounced *In Vivo* Effects

Through the resolution of pathogenesis in a titratable and re-dosable fashion

Broadly Applicable

Can achieve gene disruption/repair across multiple species and industries

In Vivo Editing Pipeline for Rare and Common Diseases

- Stealth Editors™ are likely safer and have more durable solutions for *in vivo* applications
- *In vivo* solutions are likely more cost-effective and safer than *ex vivo* solutions

	Program	Target	Approach	Discovery	Preclinical	Clinical
Liver	Alpha-1 anti-trypsin disease	*SERPINA1*	Repair the PiZ mutation (E342K) of *SERPINA1* to increase serum levels of alpha-1-antitrypsin to address emphysema and liver disease		Prevalence ~33:100,000[1]	
Liver	Undisclosed liver diseases	*Multiple*	Target selection in process			
Blood	B-thalassemia	*BCL11A*	Multi-base editing to disrupt the erythroid enhancer of *BCL11A* to induce hemoglobin switching only in red blood cells to correct the disease		Prevalence ~1:100,000[2]	
Blood	Undisclosed blood diseases	*Multiple*	Target selection in process			
	Emerging	*Multiple*	Various human diseases and agricultural applications are being evaluated to identify uniquely addressable pipeline programs			

Ongoing target selection process: low competitive landscape, human genetic data, degree of unique ability to address causal mutation, availability of biomarker, short-term clinical endpoints, prevalence, unmet need, and *ex vivo* editing hits

[1]Brode SK, Ling SC, Chapman KR. CMAJ. 2012 Sep 4;184(12):1365-71; [2]https://rarediseases.org/rare-diseases/thalassemia-major/

Stealth Editors™ are Fully Synthetic and Non-Immunogenic

Peptide nucleic acids (PNAs) are modified to strand invade dsDNA in a highly selective way to form stable PNA-DNA complexes

Oligo donors (ODNs) are single-strands of DNA modified to eliminate immune responses and contain the correction

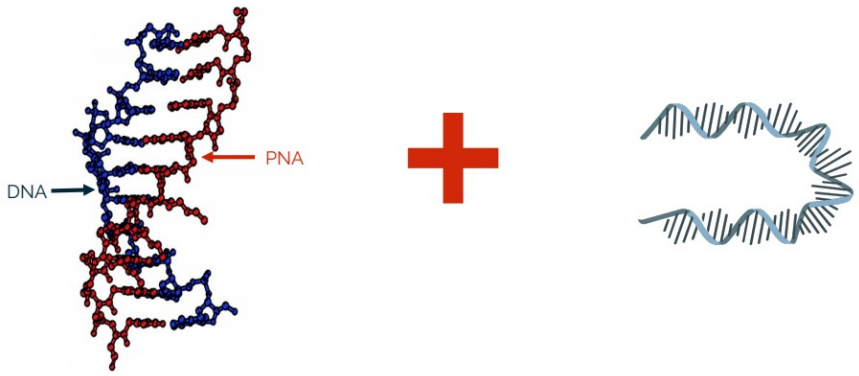
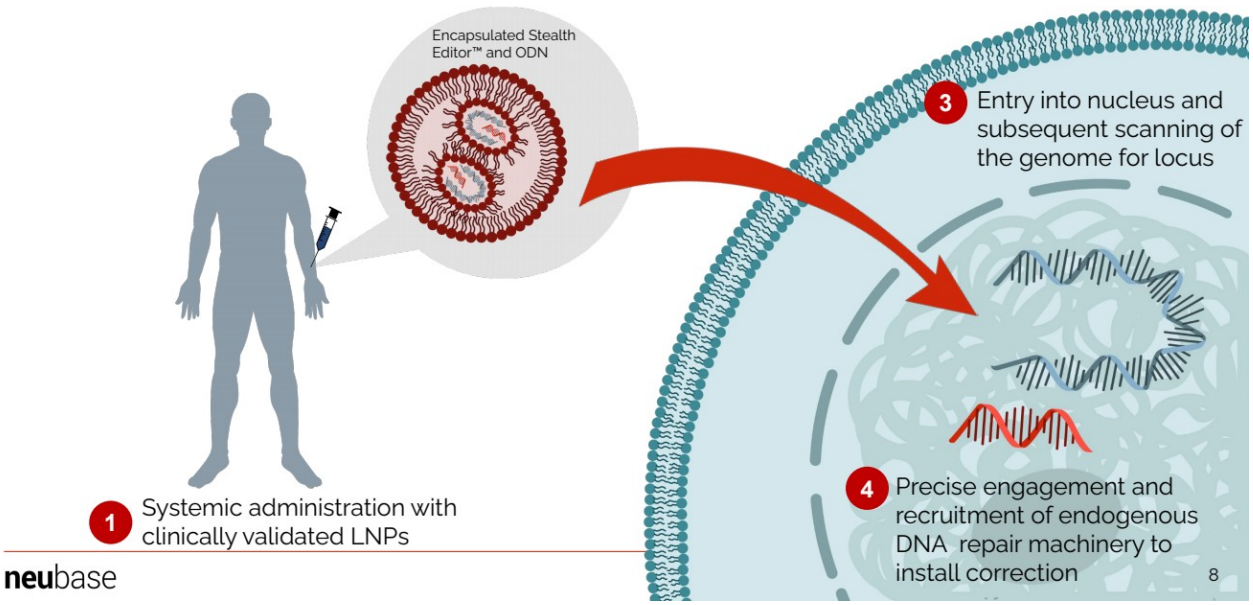


Image credit PNAS 2003, 100 (21), 12021

How Stealth Editors™ Work



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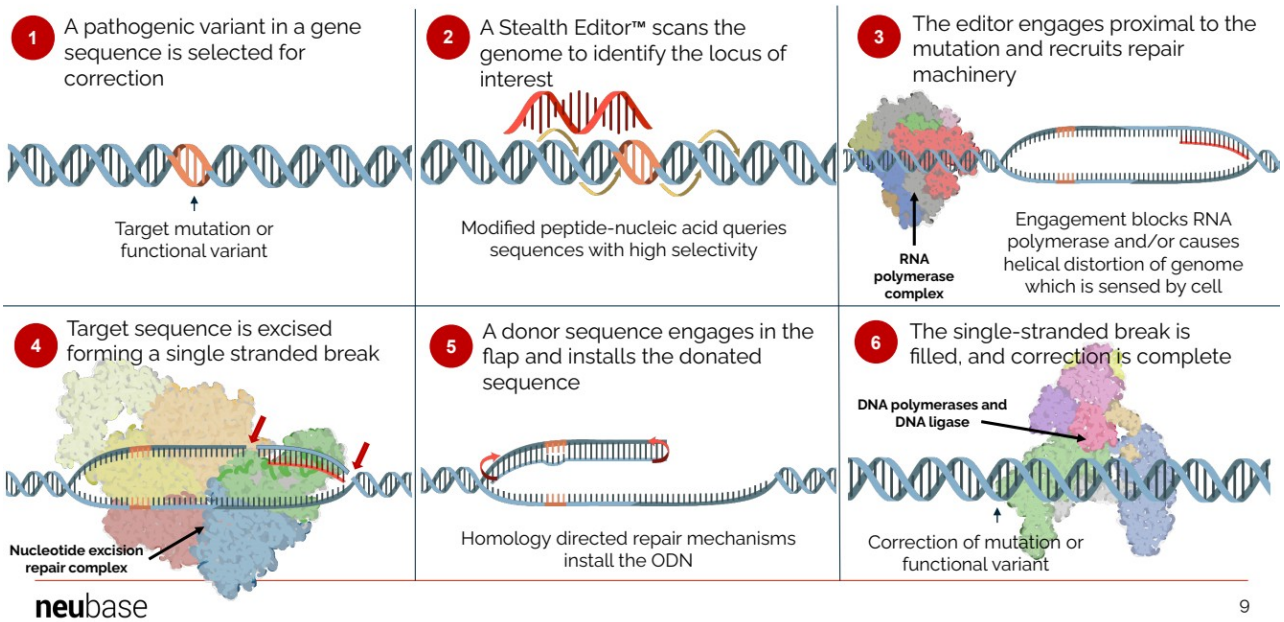
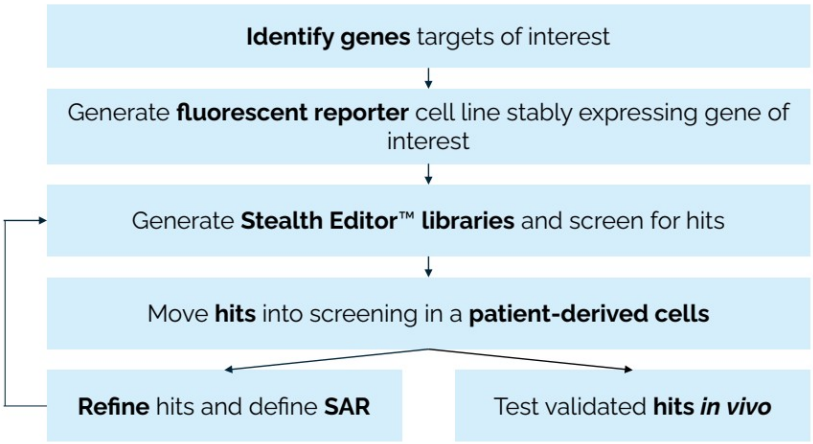
How Stealth Editors™ Work

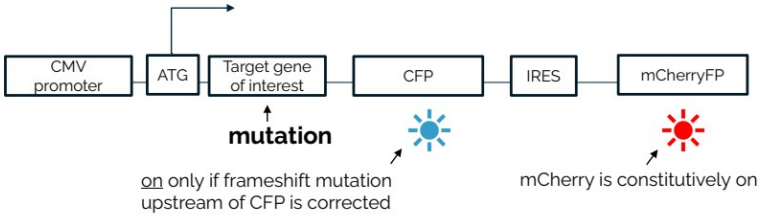


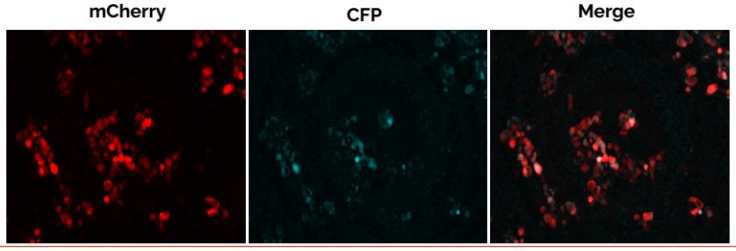
Realizing the Potential of Stealth Editing™

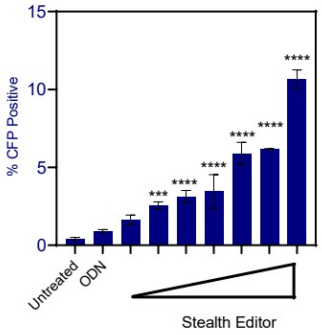


Rapid Target to Hit in Human Cells *Ex Vivo* in a Fluorescent Reporter System

Domain structure of fluorescent reporter[1]





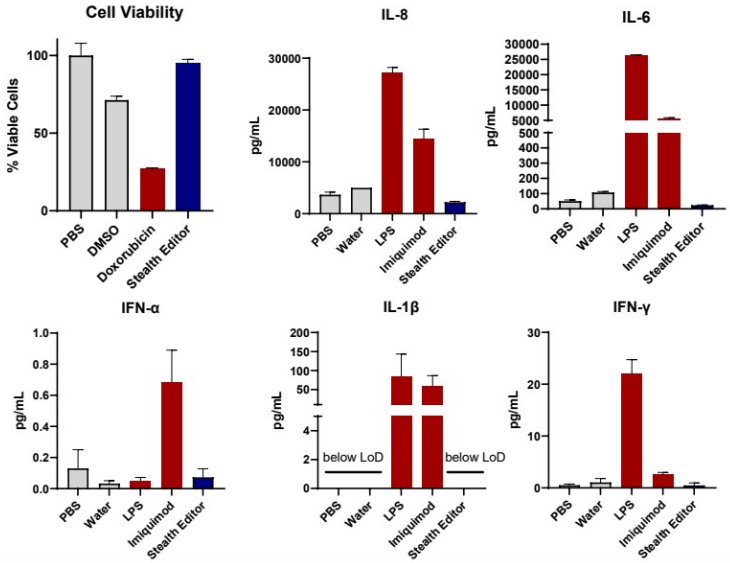


Stealth Editors™ are titratable, and editing efficiency is in-range for clinical benefit in various conditions and continues to increase with optimization

Graph represents the mean ±SD. Statistics were calculated using a one-way ANOVA with a multiple comparison post-hoc test to the untreated control. ***, $p < 0.001$; ****, $p < 0.0001$; [1]Kumar A, *et al.* BMC Genomics. 2019 Jul 24;20(1):609.

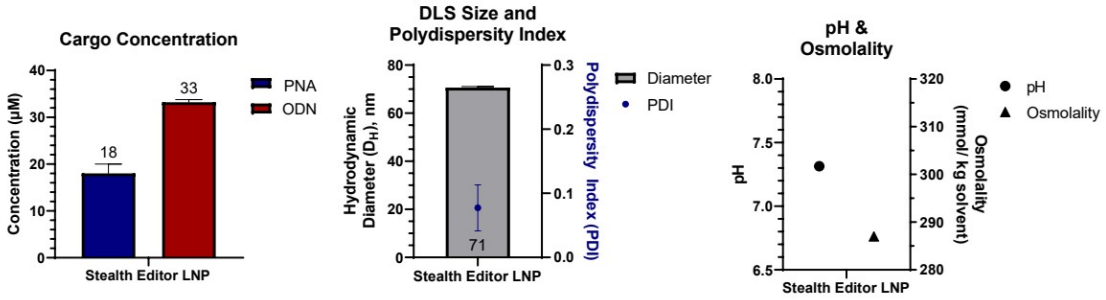
Stealth Editors™ are Non-Immunogenic in Human PBMCs

Stealth Editors™ don't affect cell viability and are non-immunogenic as measured by a cytokine panel



Data is representative of a panel of donors
Immune activator positive control LPS = 5 ng/mL
Immune activator positive control Imiquimod = 4uM
Stealth Editor = 5 uM

Stealth Editors™ are Delivered via Non-Immunogenic LNP Delivery Technology



Proprietary PNA chemistry enables co-encapsulation with ODN inside LNPs

- D_H = 71 nm
- PDI < 0.1
- pH = 7.3
- Osmolality = 287 mmol/kg solvent


neubase
www.neubasetherapeutics.com
Investor contact: Dan Ferry (daniel@lifesciadvisors.com)

Exhibit 99.2

NeuBase Announces Positive Preclinical Data Supporting the Capabilities of Stealth Editors™

- *Data show that Stealth Editors™ achieve ex vivo gene editing using simple synthetic reagents to harness the cell's own high-fidelity editing machinery*

- *Data show that Stealth Editors™ do not elicit a cell-based immunity and promise a non-immunogenic in vivo solution*

- *Data show that Stealth Editors™ can be encapsulated via established lipid nanoparticle technologies promising a non-immunogenic delivery solution and a de-risked pathway to address therapeutic targets in the liver*

- *Data featured in an oral presentation at the American Society of Gene & Cell Therapy ("ASGCT") 2023 Annual Meeting*

- *Company to present additional data showcasing gene editing capabilities of Stealth Editors™ throughout remainder of 2023, including initial non-human primate and mouse data*

- *Conference call and webcast scheduled for Monday, May 22, 2023, at 8:30 am ET*

PITTSBURGH, May 22, 2023 -- 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 announced a broad set of preclinical safety and efficiency data for its Stealth Editors development program that demonstrate the ability to achieve gene editing with a non-immunogenic system.

These preclinical data were featured in an oral presentation by Dr. Dani Stoltzfus, Vice President of Research at NeuBase, titled, "Nuclease-Free Gene Editing with Peptide Nucleic Acids: A New Class of *In Vivo* Gene Editors," at the ASGCT 2023 Annual Meeting.

"We are excited to announce preclinical data from our Stealth Editors development program that were presented during the ASGCT 2023 Annual Meeting. These data support our novel *in vivo* gene editing approach that allows 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 high-fidelity non-immunogenic approach is expected to offer advantages compared to modified bacterial CRISPR/Cas enzymes for *in vivo* gene editing applications from a safety and durability of effect perspective," stated Dietrich A. Stephan, Ph.D., Founder and Chief Executive Officer of NeuBase. "We continue to rapidly advance the development of our Stealth Editors platform, which we believe has the potential to generate best-in-class, next-generation therapies capable of addressing diverse types of high-value genetic mutations. Throughout the remainder of 2023, we

expect to announce additional preclinical data from several ongoing and planned studies in non-human primates and mice."

"Taken together, these data indicate that our novel synthetic gene editing technology is promising and can be encapsulated into delivery technologies that have delivered payloads to patients in the marketplace with known performance characteristics, enabling us to have a risk-off stance to delivery. Furthermore, the data presented at the ASGCT 2023 Annual Meeting illustrates that we can edit genes, the non-immunogenic nature of our editing system, and the exquisite fidelity of human DNA repair enzymes, all of which are the essential ingredients for a winning *in vivo* editing solution," concluded Dr. Stephan.

Overview of Preclinical Studies and Results Presented at ASGCT 2023 Annual Meeting:

- ***Ex vivo* editing with Stealth Editors** - The Company investigated the capabilities of a new editing system to effectively edit human cells *ex vivo*. The editing system is comprised of two synthetic reagents: a modified peptide nucleic acid and an oligonucleotide donor molecule. Human cells were modified to contain a fluorometric reporter system that allows rapid and real-time colorimetric readout of correction of a frameshift mutation in the genome. The result of the studies showed a dose-dependent increase in correction of the gene mutation based on expression of the newly functional fluorescent protein compared with various controls, highlighting a titratable increase in efficiency with which the Stealth Editors can engage the genome to harness the cell's own machinery to correct the mutation. The Company has made strides toward increasing editing efficiency over the past months and believes efficiency will continue to increase with technical improvements. This *ex vivo* fluorometric system is rapidly modifiable to include any gene editing target of interest and allow rapid screening against diverse targets to identify hits, supporting both the Company's emerging in-house pipeline of therapeutic programs, which the Company plans to announce later this year, as well as potential research partnerships.

- **Proven Non-Immunogenic profile in human peripheral blood mononuclear cells ("PBMCs")** - The Company demonstrated the stealth nature of its editing solution with PBMCs from multiple donors treated with either known immunostimulants or Stealth Editors packaged inside a lipid nanoparticle. The results of this study show PBMCs treated with a Stealth Editor did not impact cell viability. In addition, there was no cellular immune response for the Stealth Editor-treated cells compared with the control (PBS) across the five cytokines measured, in direct juxtaposition to the positive controls, which elicited a strong cytokine response. The conclusion from these data is that Stealth Editors do not elicit innate immune responses in the encapsulated format in which they would be administered systemically, and the Company believes this is likely to be an important differentiator of its technology when transitioned to *in vivo* gene editing.

- **Delivered via Non-Immunogenic Delivery Technology** – Using clinically-validated technology, the Company has generated Stealth Editor lipid nanoparticles (LNP) where the hydrodynamic diameter is approximately 70 nm, and the polydispersity index is <0.1. Furthermore, the Company's drug product has a physiologically acceptable pH of 7.3 and

an osmolality in the range of 300. These data demonstrate that NeuBase has overcome the challenge of how to encapsulate Stealth Editors and sets the stage to move this program into *in vivo* studies.

Myotonic Dystrophy Type 1 (DM1) Program Data at ASGCT 2023 Annual Meeting

In addition, during the ASGCT 2023 Annual Meeting, the Company presented data from its Myotonic Dystrophy Type 1 (DM1) program in an oral presentation titled, “Toxicology, Pharmacokinetics and Biodistribution of a PATrOL™-Enabled Investigational Genetic Therapy for Myotonic Dystrophy, Type 1.” The slide presentations for the Stealth Editors™ and DM1 data updates at the ASGCT 2023 Annual Meeting will be made available on the Publications and Presentations section of the NeuBase website (click here).

Conference Call and Webcast

The Company’s management team will host a conference call and webcast with investors and analysts to discuss the data presented at the ASGCT 2023 Annual Meeting on **Monday, May 22, 2023, at 8:30 am ET**. The live call may be accessed by dialing (877) 407-9208 for domestic callers and (201) 493-6784 for international callers and entering the conference ID: 13738958. The live webcast presentation with accompanying slides will be accessible here and on the Investor Relations Calendar page of the Company’s website at https://ir.neubasetherapeutics.com/news-events/ir-calendar. Following the completion of the event, a replay will be available on the Company’s website.

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 and the Company’s statements regarding potential collaborations. 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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