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): April 1, 2020

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.)

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

(646) 450-1790
(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.

On April 1, 2020, NeuBase Therapeutics, Inc. (the "Company"), updated its corporate presentation materials to include information from its webcasted conference call held on March 31, 2020 and the target milestones of its two lead programs utilizing its PATrOL™ platform: the NT0100 Program focused on the treatment of Huntington's Disease and the NT0200 Program focused on the treatment of Myotonic Dystrophy Type 1. A copy of the updated corporate presentation materials referred to in this Item 7.01 of Current Report on Form 8-K (this "Current Report") is furnished herewith as Exhibit 99.1 to this Current Report and incorporated herein by reference.

Safe Harbor Statement under the Private Securities Litigation Reform Act of 1995

This Current Report contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act. These forward-looking statements include, among other things, statements regarding the Company's target milestones for its two lead programs. 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 Current Report. Factors or events that we cannot predict, including those described in the risk factors contained in our filings with the U.S. Securities and Exchange Commission, 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 Current Report, including: the Company's plans to develop and commercialize its product candidates; the Company's plans to commence clinical trials in Huntington's disease and myotonic dystrophy type 1 and to potentially expand the pipeline into other indications; the utility of the preclinical data generated in existing studies performed by the Company in determining the results of potential future clinical trials and of the potential benefits of the PATrOL™ platform technology; the timing of initiation of the Company's planned clinical trials; the timing of the availability of data from the Company's clinical trials; the timing of any planned investigational new drug application or new drug application; the Company's plans to research, develop and commercialize its current and potential future product candidates; the clinical utility, potential benefits and market acceptance of the Company's current and potential future 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 in our filings with the U.S. Securities and Exchange Commission. 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.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Corporate Presentation Materials.

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: April 3, 2020

By: /s/ Sam Backenroth
Sam Backenroth
Chief Financial Officer

Exhibit 99.1

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

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," 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 NeuBase's plans to develop and commercialize its product candidates, including NT0100 and NT0200; the timing of initiation of NeuBase's planned clinical trials; the timing of the availability of data from NeuBase's clinical trials; the timing of any planned investigational new drug application or new drug application; NeuBase's plans to research, develop and commercialize its current and future product candidates; the clinical utility, potential benefits and market acceptance of NeuBase's product candidates; NeuBase's commercialization, marketing and manufacturing capabilities and strategy; NeuBase's ability to protect its intellectual property position; and NeuBase's estimates regarding future revenue, expenses, capital requirements and need for additional financing and the impact of COVID-19 on us and our partners. 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.

THE NEXT GENERATION OF ANTISENSE THERAPY

World-class science and drug developers

- **Designed to be a first-in-class platform with unique advantages; originally developed at Carnegie Mellon University (CMU)**
- 16 patents and patent applications describing composition of matter & field of uses of platform provides protection to between 2034 and 2040, excluding available extensions
- Leadership team has led the development of drugs that are in the market helping patients today and includes recognized scientific thought leaders

Key advantages over other ASO[1] technologies

- **We believe our pharmacokinetic, pharmacodynamic and tolerability data demonstrates PATrOL™ as a viable therapeutic modality**
- Our compounds target pre-mRNA, mRNA and double-stranded RNA targets

Initial focus on monogenic disorders

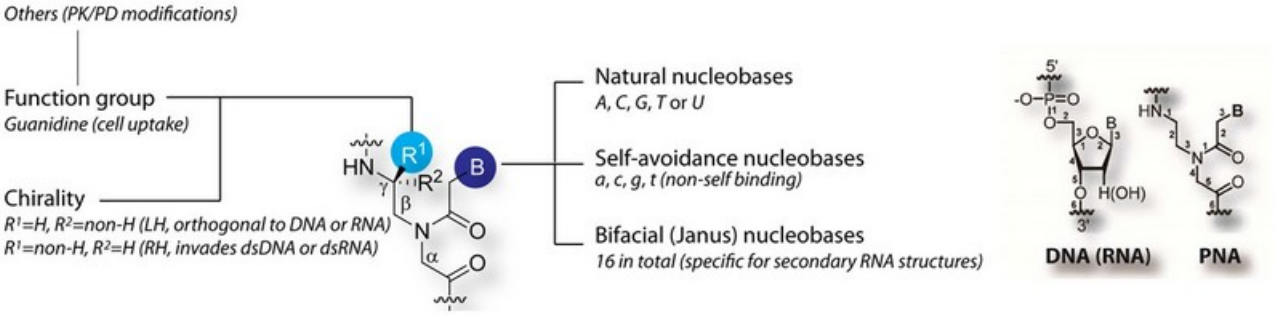
- **Ongoing development for Huntington's disease and myotonic dystrophy type 1 with potential billion-dollar peak sales opportunity in each indication[2]**
- Potential to leverage development costs for first programs to address other repeat expansion diseases

Ability to expand into additional indications

- **Pharmacokinetics, pharmacodynamics and tolerability profiles coupled with modular platform focused in orphan disorders portend a scalable pipeline**
- Pharmacokinetic data enables expansion into therapeutic areas others cannot address

[1]antisense oligonucleotide, [2]based on prevalence and market dynamics for RNA therapeutics

THE PATROL™ BACKBONE CHEMISTRY



ENTERING THE ERA OF SCALABLE DRUG DEVELOPMENT

Peptide-Nucleic Acid Antisense Oligos (PATrOL™)

"Digital" design of compounds to complement target RNA sequence vs. "analog" library screening

nucleobases A,C,G,T, U, engineered mono- and bi-facial

- hydrogen bonding in a complementary manner to target RNA
- mechanism of action is to inhibit translation or alter splicing
- modulate binding affinity to target mutant positions
- bind double-stranded RNA targets

synthetic charge-tunable peptide backbone

- higher binding affinity to target than standard ASO technologies
- pre-organized backbone simplifies manufacturing
- resistant to degradation by nucleases & proteases
- well tolerated *in vivo* with no immune response

targeting technology

- broad biodistribution after systemic administration
- integrated targeting technology into backbone
- cell internalization by multiple mechanisms
- flexibility to modify with ligands and antibodies

linker technology for template directed self-assembly

- short compounds bind stably across expansions

POTENTIAL ADVANTAGES OF PATROL™ OVER EARLY ASOS

COMPOUND PROPERTY	ASOS	NEUBASE	NEUBASE ADVANTAGE
Modular molecular design	✗	✓	Tunable features of backbone enables applications in a potentially scalable manner
Proprietary engineered nucleobases	✗	✓	Tuning nucleobase binding affinity to improve selectivity and minimize off-target effects
Favorable length (3-mer+)	✗	✓	Compounds (oligos) can be made shorter when coupled to bi-facial nucleobases and thus potentially better diffuse through tissue
Ability to open up and bind to double-stranded RNA	✗	✓	Higher selectivity for mutant allele if aberrant secondary structure is formed; potentially more rapid design and screening as secondary structures are not limiting
No self-aggregation	✗	✓	No self-aggregation of drug via engineered "self avoidance" nucleobases
Innately stable to degrading enzymes	✗	✓	Stable in the body; resistant to degradation by nucleases and proteases
Cell membrane permeability and water soluble	✗	✓	Portion of compound potentially directly reaches the intracellular target in cytoplasm and nucleus; portion of dose likely endocytosed; initial PNAs were not water soluble
Broad biodistribution without ligands or LNPs[1]	✗	✓	Every compound backbone is covalently modified with targeting technology which allows the compound to penetrate into potentially all cell types
No innate or adaptive immune activation	✗	✓	No neutralizing antibodies, pro-inflammatory cytokines or type 1 interferon production seen to date
Capable of transcriptional silencing and modifying transcriptional splicing	✗	✓	Potential to address dominant and recessive genetic diseases through enhanced steric hindrance of ribosome or spliceosome displacement
Able to target genomic double-stranded DNA	✗	✓	Future opportunities in gene editing and transcriptional regulation possible

[1]lipid nanoparticle formulations

PLATFORM VALIDATION: KEY ADVANTAGES

Pharmacokinetics

Do our compounds achieve **broad biodistribution** after systemic delivery?

Yes: Broad distribution at therapeutically relevant levels[1] after single-dose IV administration in non-human primates

Pharmacodynamics

Do our compounds show **activity** in silencing disease-causing mutations?

Yes: Enrichment of mutant huntingtin protein knock-down relative to normal protein in Huntington's disease

Tolerability

Are our compounds well **tolerated** in cell and animal studies?

Yes: Generally well tolerated *in vivo* in both non-human primates and rodents and low cytotoxicity across candidates *in vitro*

PATrOL™ technology is a first-in-class platform with key advantages over early ASO technologies

[1]compound levels in tissues show activity in patient-derived cell lines

POTENTIAL ADDITIONAL NEWS FLOW: TARGET MILESTONES[1]

NT0100 Program

Huntington's Disease

Lead Candidate Selection	YE 2020
Begin IND-Enabling Studies	H1 2021
IND Filing	YE 2021
Clinical Data	YE 2022

NT0200 Program

Myotonic Dystrophy Type 1

Lead Candidate Selection	H1 2021
Begin IND-Enabling Studies	H2 2021
IND Filing	H2 2022

Pipeline Build Out

Potential Additional Pipeline Programs Being Prioritized Based on NHP PK Roadmap

[1]all timelines reference calendar years not fiscal years

EARLY ASO[1] TECHNOLOGIES HAVE LIMITATIONS

Pharmacokinetics

Other ASO technologies are not broadly biodistributed after systemic administration often requiring local delivery (for example via intrathecal administration for central nervous system (CNS) disorders)

Pharmacodynamics

Other ASO technologies have difficulty discriminating between mutant and wild type alleles, and generally inhibit both equally

Tolerability

Other ASO technologies have shown toxicity when systemically administered including proinflammatory effects (vasculitis/inflammatory infiltrates), nephrotoxicity and hepatotoxicity unrelated to lysosomal accumulation and thrombocytopenia[2]

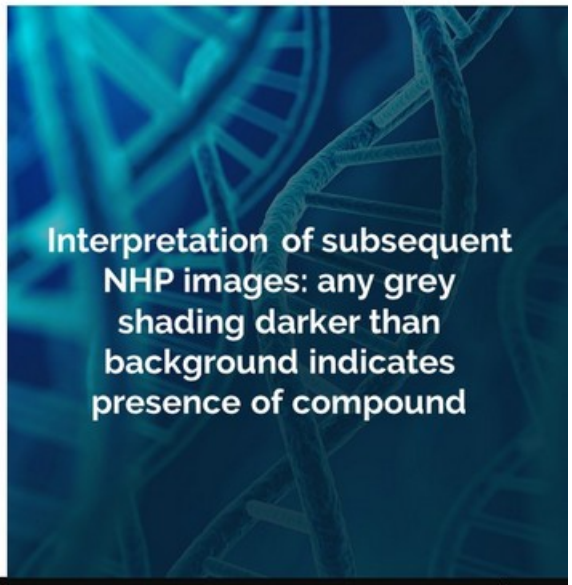
The PATrOL™ technology is built upon three decades of innovation in the field

[1]antisense oligonucleotide; [2]https://doi.org/10.1177/0192623314551840


PHARMACOKINETICS
Broad biodistribution after systemic delivery

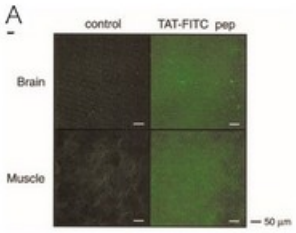
NON-HUMAN PRIMATES (NHP): PHARMACOKINETICS STUDY DESIGN

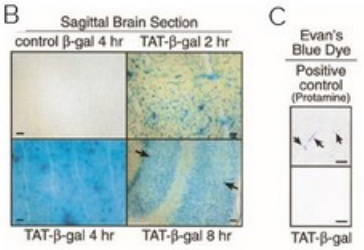
- *Macaca fascicularis* NHPs were selected as model system
- 5 mg/kg of a Huntington's disease candidate compound was injected into the tail vein of NHPs
- NHPs were sacrificed and flash-frozen at 4 hours, 12 hours and 7 days post-injection
- Plasma, whole blood, urine, and feces were collected and assayed
- Animals were sectioned to 40µm and imaged via quantitative whole-body autoradiography (QWBA)
- A concentration standard was run for every image

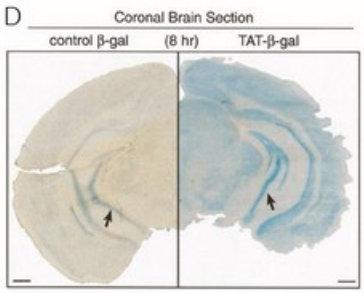


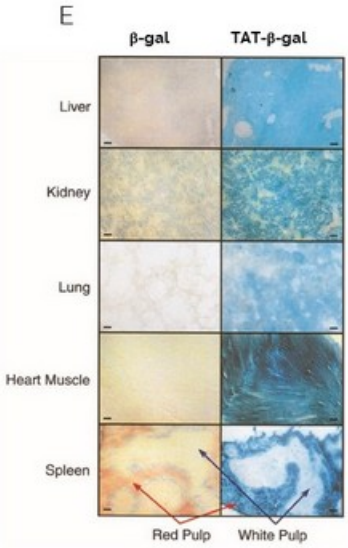
HIV TAT ORIGINS OF PATROL™ TARGETING CHEMISTRY

Derived from HIV TAT Peptide Which Traffics Large Payloads Across the Intact BBB









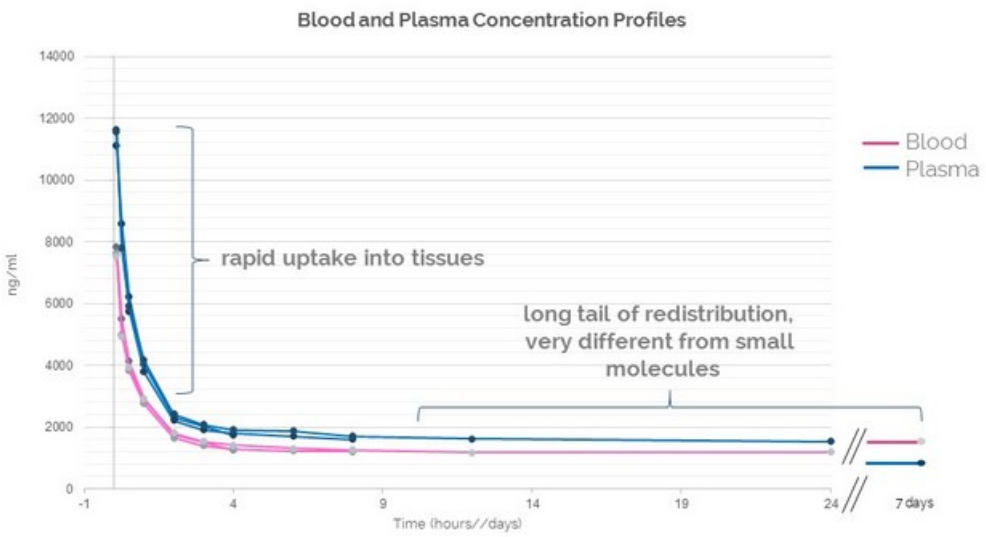
A. Fluorescence confocal microscopy of hemispheric sagittal brain sections (top) and skeletal muscle tissue sections (bottom) isolated from mice 20 min after intraperitoneal (ip) injection as in (A). Scale bars, 100 mm. **B.** β-Gal activity (X-Gal staining) in hemispheric sagittal brain sections from mice injected ip with TAT–β-Gal or control β-Gal; sections were made after the indicated times. Eight hours after ip injection, TAT–β-Gal localized primarily to the nuclei of cell bodies (arrows) throughout the brain section. Sections were developed in X-Gal for 16 hours. Scale bars, 100 mm. **C.** Extravasated Evan's blue dye was detected adjacent to blood vessels throughout the brain sections (50 mm) from mice treated with the protamine positive control (top) but was not detected in sections from TAT–β-Gal– treated mice (bottom). Scale bars, 100 mm. **D.** β-Gal activity in a low-magnification coronal brain section from mice 8 hours after ip injection with control β-Gal (left) or TAT-β-Gal (right), showing activity through-out the brain. Arrows indicate the region of endogenous β-Gal activity. Scale bars, 500 mm **E.** Transduction of TAT–β-Gal into mice. Analysis of β-Gal enzymatic activity assessed by X-gal staining in the liver, kidney, lung, heart muscle, and spleen is shown. Samples were analyzed 4 hours after ip injection of proteins, except for kidney tissue, which was analyzed 8 hours after injection. There is weak β-Gal activity in the white pulp areas of the spleen. Sections were developed for 4 hours. Scale bars, 100 mm.

Schwartze SR et al. In Vivo Protein Transduction: Delivery of a Biologically Active Protein into the Mouse. Science 1999; 285: 1569-72.

NHP DATA: RAPID UPTAKE OUT OF CIRCULATION AFTER IV ADMINISTRATION

Half-life in plasma of approximately **1.5 hours**

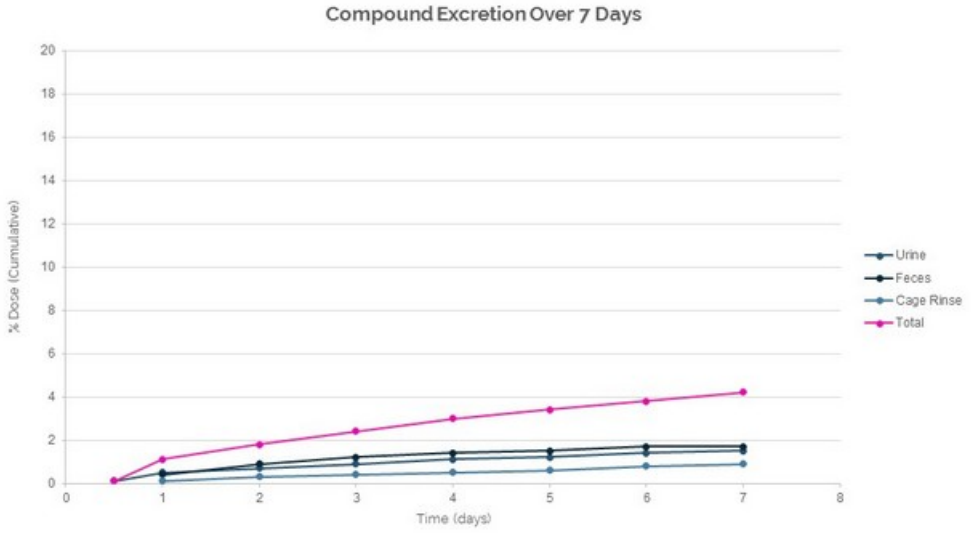
Long tail likely **redistribution** and slow **excretion** of **intact compound**



NHP DATA: DURABLE RESIDENCE TIME IN TISSUES WITH SLOW ELIMINATION

1.5% of the administered drug was excreted in the urine over a **7-day** period

1.7% of the administered drug was excreted in the feces over a **7-day** period



PATROL™ PENETRATES INTO EVERY ORGAN SYSTEM AFTER IV ADMINISTRATION

1 Retina
2 Trachea
3 TA
4 Stomach
5 Brain

6 Putamen
7 Caudate
8 Substantia Nigra
9 Lateral Ventricle

10 Cortex
11 Spinal cord
12 Skeletal muscle
13 Lung
14 Bladder

15 Olfactory bulb
16 Cerebellum
17 Kidney medulla
18 Intestine
19 Heart (myocardium)

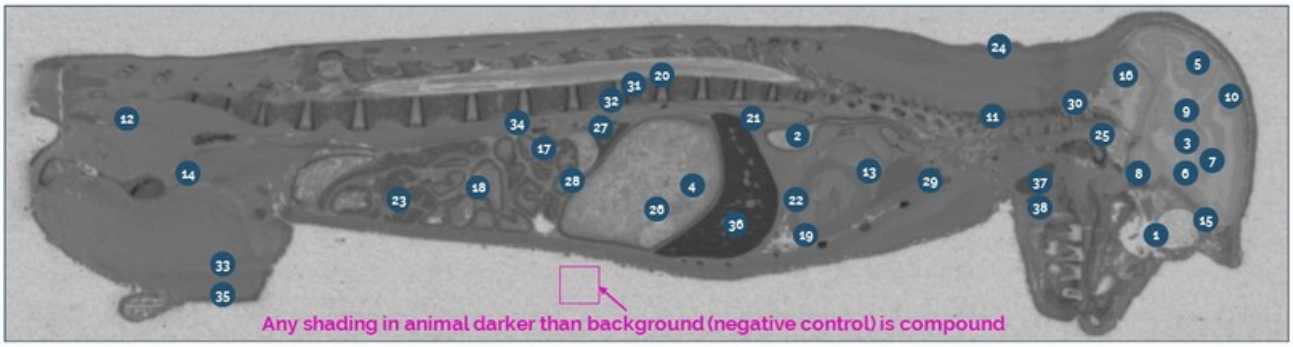
20 Spinal cord
21 Aorta
22 Cardiac blood
23 Mesenteric lymph nodes
24 Skin

25 Pituitary
26 Spleen
27 Adrenal gland
28 Pancreas
29 Thymus

30 Parotid
31 Bone marrow
32 Cartilage
33 Bone
34 Kidney Cortex

35 Cartilage (joint)
36 Liver
37 Salivary glands
38 Lymph nodes

12 µM
600 nM
20 nM

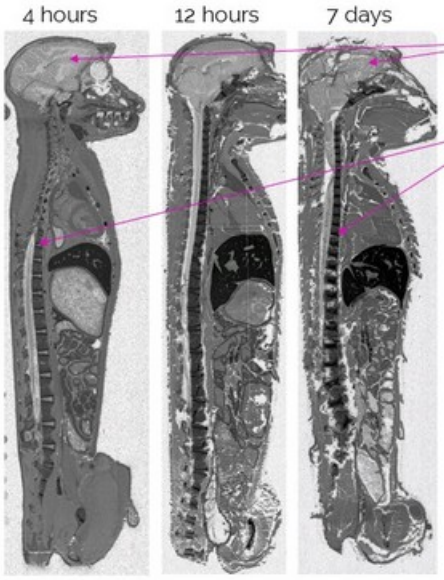


[1]40µm section through midsagittal plane at 4 hours post-single dose injection

NHP: RAPID UPTAKE AND DURABILITY AFTER SYSTEMIC INJECTION

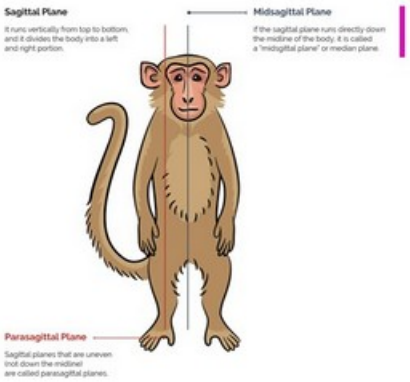
At various time points post dosing, animals were sectioned along the **midsagittal plane** for analysis

Shading above background reflects **rapid uptake in multiple organs** as early as **4 hours** and increased uptake over time



Brain enrichment over 7 days

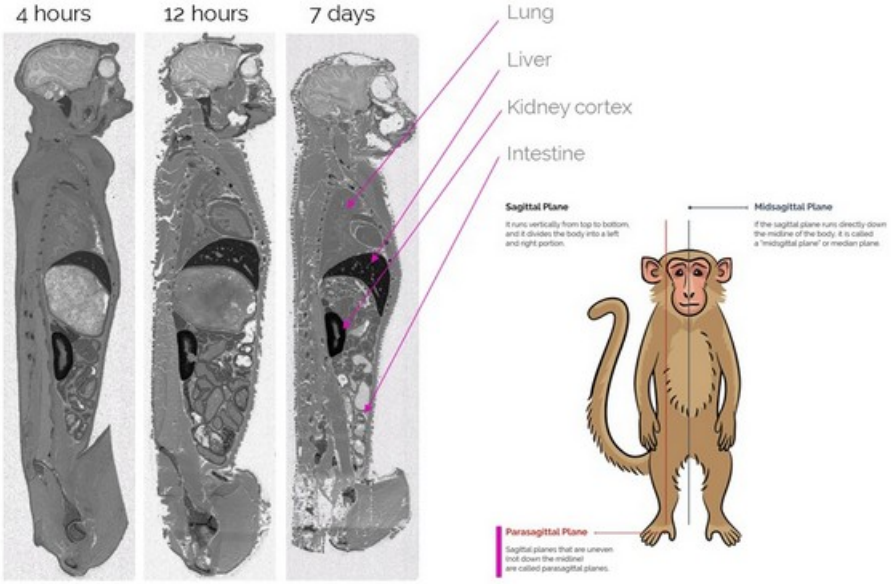
Bone enrichment over 7 days



NHP: RAPID UPTAKE AND DURABILITY AFTER SYSTEMIC INJECTION

Parasagittal plane shows compound **resides in the body and redistributes** over 7 days after systemic administration

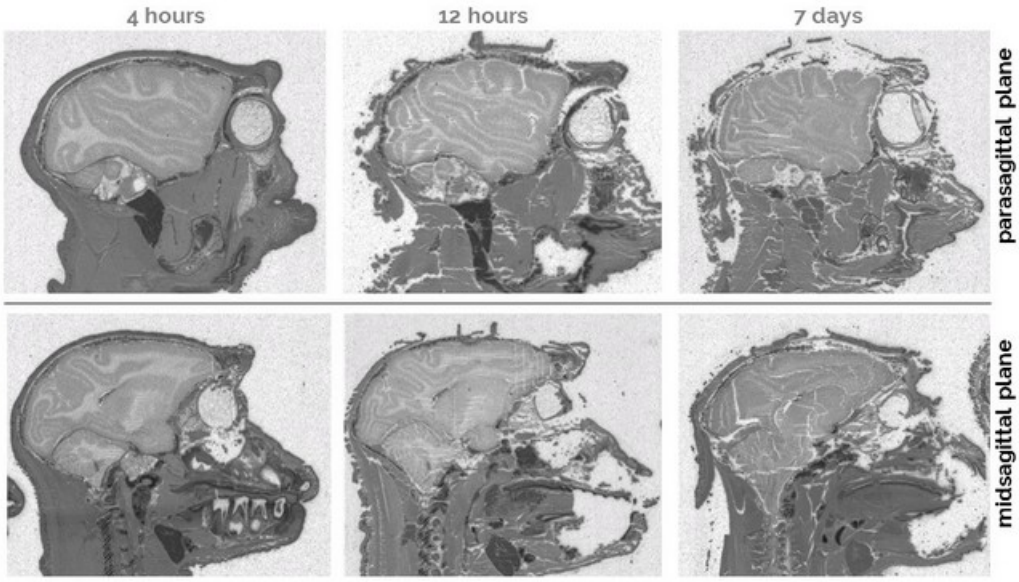
Shows **consistently high concentrations** in brain, muscle, liver, kidney, lung, among other organs



NHP: INCREASE IN DISTRIBUTION TO KEY BRAIN REGIONS OVER 7 DAYS

Through redistribution, concentrations of compound **increase by up to ~2x** in the CNS over 7 days[1]

Compound redistributes to **more homogeneous levels** across cortical and white matter structures

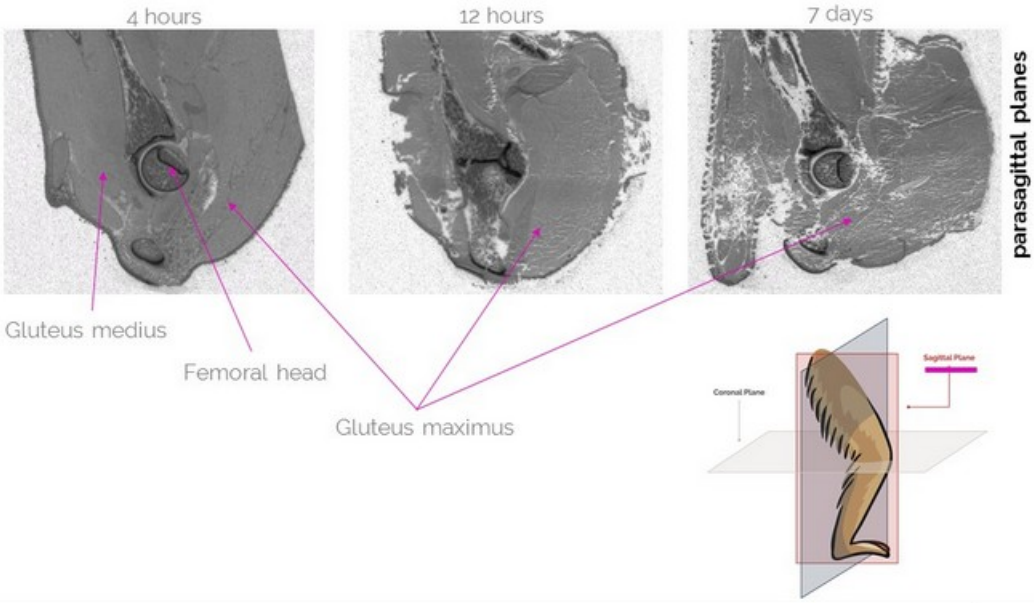


[1]7 days vs. 4 hours; latest timepoint tested

NHP: DURABLE CONCENTRATIONS IN SKELETAL MUSCLE OVER 7 DAYS

~90% of the amount of compound present at 4 hours is present at 7 days[1]

Skeletal muscle distribution **remains high** over the 7 day time course



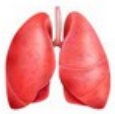
[1]latest timepoint tested

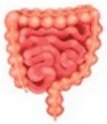
TISSUE CONCENTRATIONS OPEN BROAD PIPELINE OPPORTUNITIES

Achieves Therapeutically Relevant Levels* after Single Dose IV Administration of 5 mg/kg with Broad Biodistribution in Many Tissues

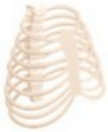

Retina


Brain & Spinal Cord


Lung


Intestine


Heart muscle


Bone


Skeletal Muscle


Spleen & Lymph Nodes

This biodistribution is also relevant in oncology for metastatic disease

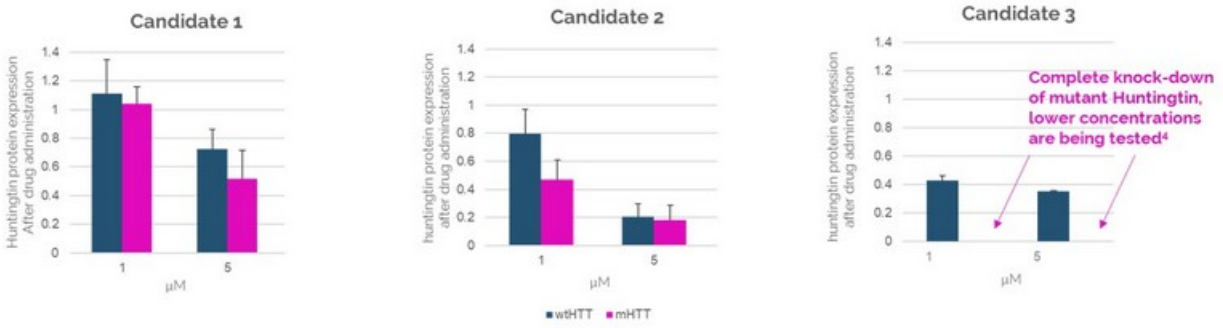
neubase

* compound levels in tissues show activity in patient-derived cell lines


PHARMACODYNAMICS
Activity in silencing disease-causing mutations

KNOCK-DOWN OF MUTANT HUNTINGTIN PROTEIN IN PATIENT CELL LINES

SAR[1] of candidate compounds targeting CAG[2] Repeats in patient HD[3] cell lines: Potency and Selectivity



Lower Selectivity
Moderate Potency

Moderate Selectivity
Moderate Potency

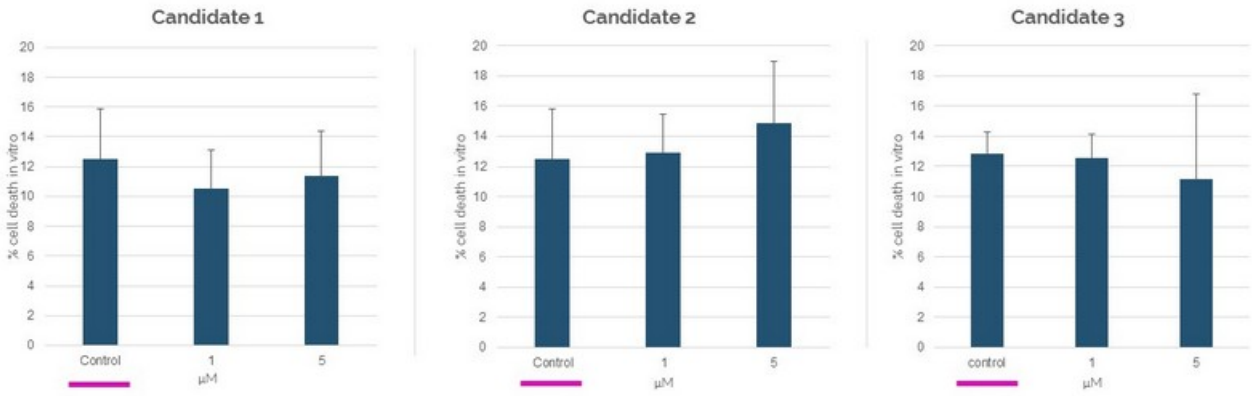
High Selectivity
High Potency

neubase

[1]structure-activity relationship; [2]cytosine-guanine-adenine; [3]Huntington's disease; [4]concentrations down to 1nM are currently being tested for potent compounds


TOLERABILITY
Well tolerated in pre-clinical studies

CANDIDATES DID NOT DEMONSTRATE CYTOTOXICITY *IN VITRO* AT ACTIVE CONCENTRATIONS



% cytotoxicity *in vitro in fibroblast cell line from patient with 151/21 repeat alleles*; cells in culture have a baseline rate of turnover as indicated by control

IN VIVO TOLERABILITY AT SINGLE AND CHRONIC SYSTEMIC DOSING

Non-Human Primates

5 mg/kg[1] single dose injections in tail vein
Were generally well tolerated

Mouse

Chronic 3x/week intraperitoneal injections of 2 mg/kg[2] for 5 weeks were generally well tolerated

[1,2]highest dose tested

THE PATH FORWARD FOR HD, DM1 AND BEYOND

Optimize

Lead compounds to increase potency through modulating binding affinity and cooperativity of self-assembly on mRNA target

Characterize

Drug residence times in excess of one week in CNS and skeletal muscle to define dosing requirements with single and multidose systemic administration

Define

The durability of effect of lead compounds in patient-derived cell lines and in genetic animal models of disease

Initiate

IND-enabling toxicology packages

Expand

Into new pipeline programs and organ systems based on NHP pharmacokinetics data and unique platform attributes including into dominant and recessive monogenic diseases and oncology

OUR DEVELOPMENT TEAM INCLUDES LUMINARIES IN BIOTECHNOLOGY

DIETRICH STEPHAN, PHD
CEO

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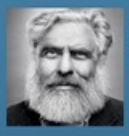

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CINRYZE (C1 esterase inhibitor [human])
CYTOGAM
Dalvance (dalbavancin) for injection 500 mg
Eraxis (anidulafungin IV)
Ethyol (amifostine)
INTELENCE (etravirine) tablets
PREZISTA (darunavir) tablets
SYNAGIS PALIVIZUMAB

STRONG IP PORTFOLIO

NeuBase Therapeutics holds an **exclusive license** to the PATrOL™ technology, with 16 patents and patent applications describing composition of matter and uses of the platform[1]

Patents have expiration dates from **2034 to 2040**, not including "Hatch Waxman" **extensions**

Developed at **Carnegie Mellon University and NeuBase**

USPTO has **allowed claims** for Composition of Matter on Janus Bases

[1]2US Patents, 9 US applications, and 5 foreign applications

PLATFORM VALIDATION: KEY ADVANTAGES

Pharmacokinetics

Broad distribution at therapeutically relevant levels[1] after single-dose IV administration in non-human primates

Pharmacodynamics

Enrichment of mutant huntingtin protein knock-down relative to normal protein in Huntington's disease

Tolerability

Generally well tolerated *in vivo* in both non-human primates and rodents and with low cytotoxicity across candidates *in vitro*

PATrOL™ technology is a first-in-class platform with key advantages over early ASO technologies

[1]compound levels in tissues show activity in patient-derived cell lines


neubase