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): December 16, 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.

Announcement of Preclinical Data

On December 16, 2020, NeuBase Therapeutics, Inc. (the "Company") conducted a webcasted conference call to discuss positive preclinical data from its therapeutic program for the treatment of Myotonic Dystrophy Type 1. The presentation materials to the conference call are furnished herewith as Exhibit 99.1 to this Current Report on Form 8-K (this "Current Report"). The conference call will be archived and accessible at https://ir.neubasetherapeutics.com/ for approximately thirty days following the conference call.

Item 8.01 Other Events.

On December 16, 2020, the Company announced preclinical data from its therapeutic program for the treatment of Myotonic Dystrophy Type 1. A copy of the press release related to these data is attached hereto as Exhibit 99.2 to this Current Report and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Preclinical Data Conference Call Presentation Materials for conference call held on December 16, 2020.
99.2	Press Release, dated December 16, 2020.

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: December 16, 2020

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

neubase

Accelerating the genetic revolution using a new class of synthetic medicines

An emerging pipeline: resolution of causality in myotonic dystrophy, type 1 (DM1) via a PATrOL™-enabled therapy

December 16, 2020

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. These forward-looking statements include, among others, those related to potential significance and implications of the Company's positive *in vitro* and *in vivo* preclinical data for its PATrOL™-enabled anti-gene therapies for the treatment of myotonic dystrophy, type 1. 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 risks that prior data will not be replicated in future studies; 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 the risk factors contained within our filings with the U.S. Securities and Exchange Commission. 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.

Agenda

The opportunity
Myotonic dystrophy, type 1 (DM1)
In vitro activity
In vivo activity (IV)
DMPK protein levels
PATrOL™ technology platform
Pipeline
The team
Summary

Take-aways

PATrOL: a validated disruptive technology

- *In vivo* data demonstrating target engagement and therapeutic activity after single IV administration
- Broad biodistribution demonstrated in NHPs
- Potential to increase, decrease, or change protein function in a single unified platform

Large markets with high unmet medical need

- Myotonic dystrophy, type 1 (DM1)
- Huntington's disease (HD)

Proven team of drug developers

Myotonic dystrophy, type 1

- A severe intractable genetic disease
- Affects >5/100,000 with large unmet need

Our data describing a solution to this disease

- Rapid induction of rescue across a multitude of mis-spliced transcripts *in vitro* and *in vivo*
- Novel delivery technology allows single-dose IV administration without conjugation to biologics
- Designed to not reduce DMPK protein
- Well tolerated at therapeutically active dose

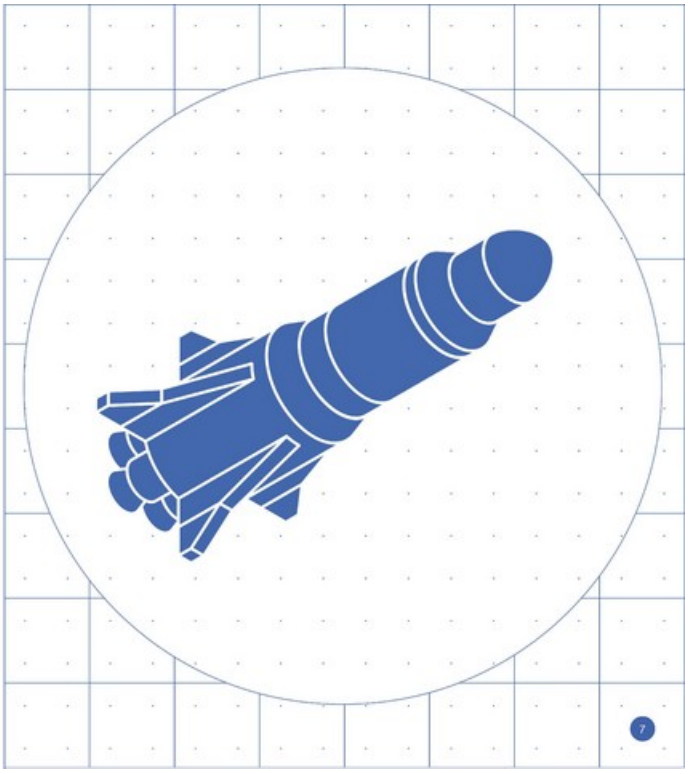
The opportunity

Knowledge-based design

We are in the midst of a transformation in the pharmaceutical industry

Targeting misbehaving genes

Most diseases have genetic drivers that result in cellular dysfunction



Many disease-causing genes are known

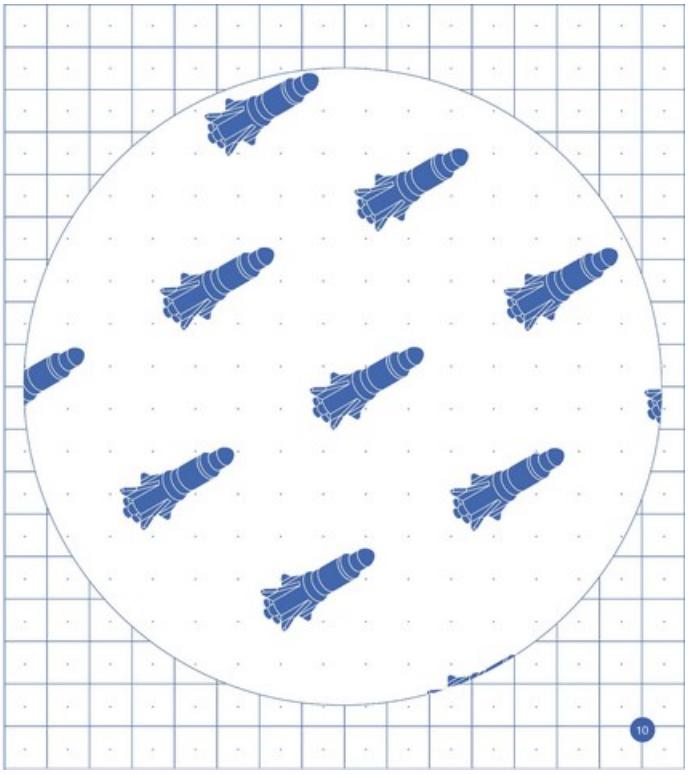
We use nature's own digital information encoding schema to custom design medicines for rare and common diseases

We can engage both DNA and RNA

With a single platform we can modulate genetic targets to resolve gain-, loss- and change-of-function mutations

Landscape transformation

A unique platform with broad applicability yet without many of the limitations of other genetic medicine approaches



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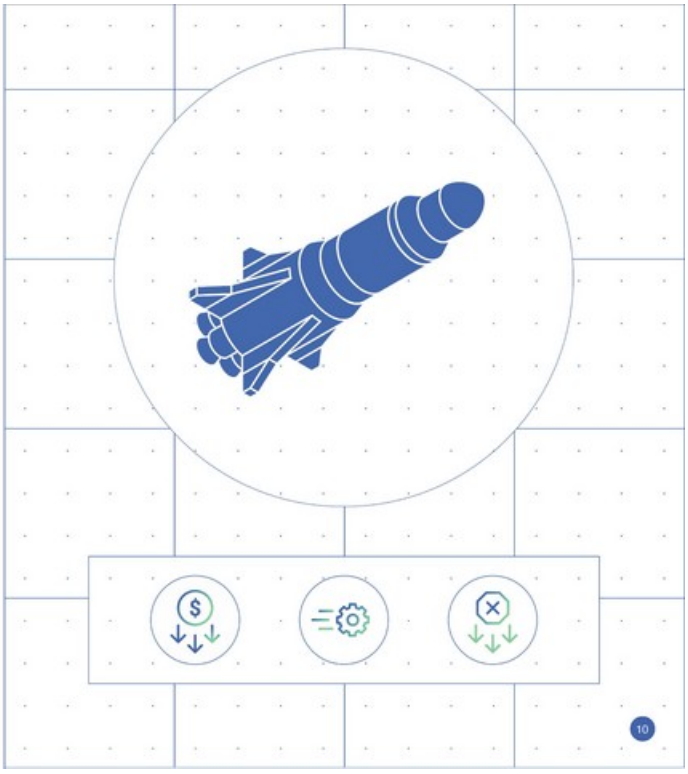

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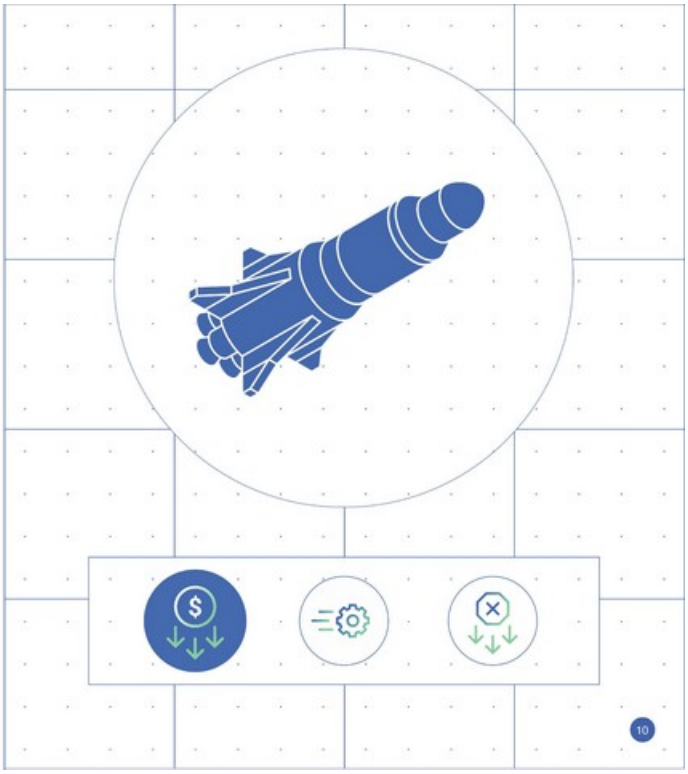

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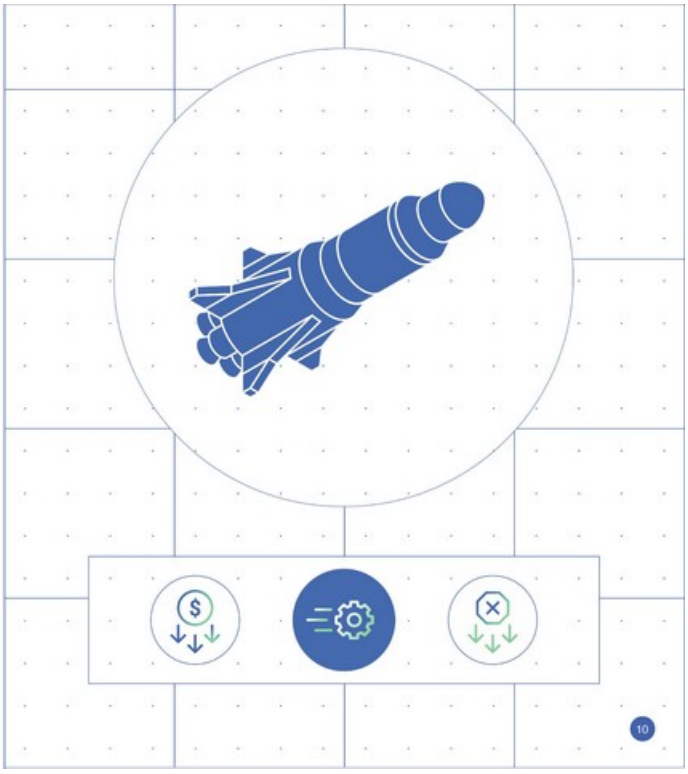


Myotonic dystrophy, type 1 (DM1)

The disease

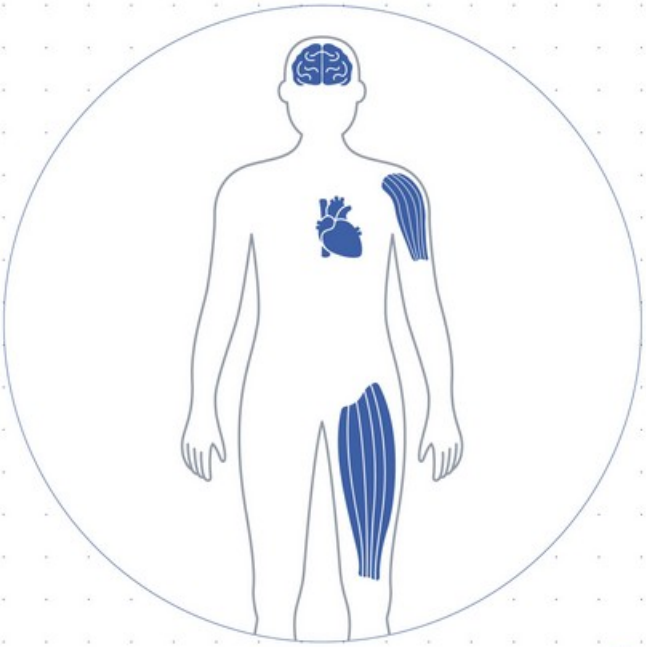
Mild DM1 is characterized by cataract and mild myotonia (sustained muscle contraction); life span is normal.

Classic DM1 is characterized by cataract and myotonia, as well as muscle weakness and wasting, and often cardiac conduction abnormalities; adults may become physically disabled and may have a shortened life span.

Congenital DM1 is characterized by hypotonia and severe generalized weakness at birth, often with respiratory insufficiency and early death; intellectual disability is common.

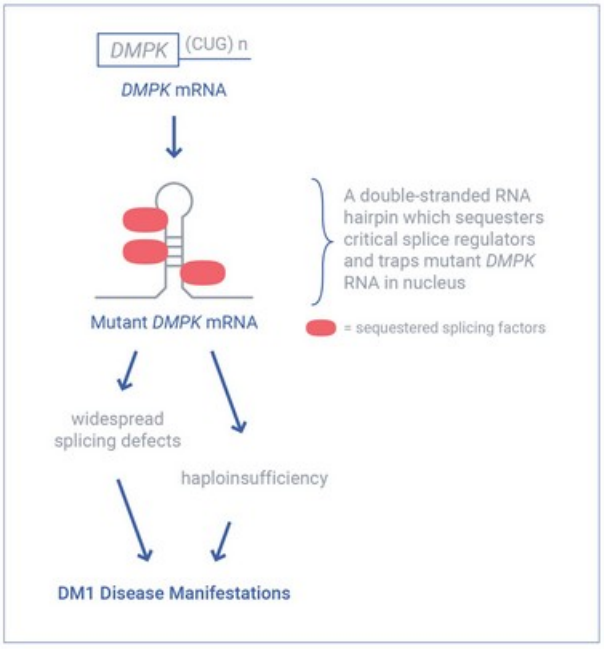
The prevalence of DM1 is >5/100,000 in the general population.

Current treatment options only address symptoms, not the disease itself.



The genetic cause

A genetic mutation results in generalized mis-splicing of many transcripts and haploinsufficiency of the *DMPK* protein which both contribute to the disease



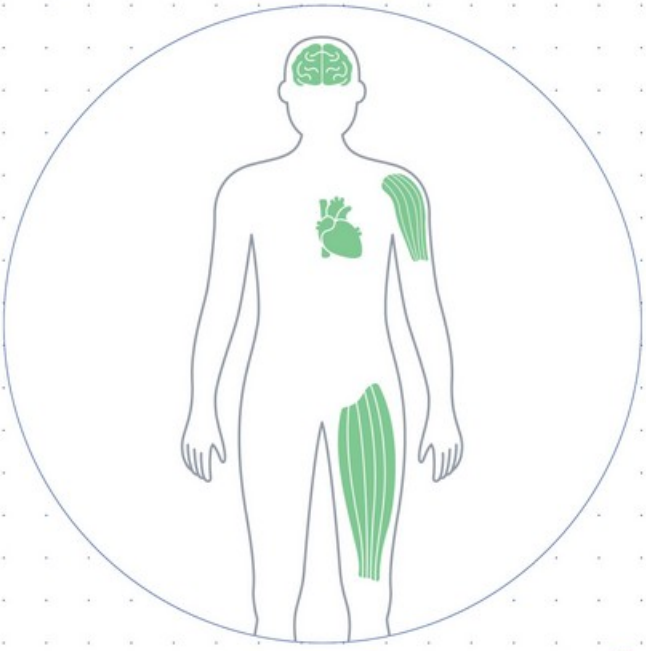
Genetic therapies fall into two categories

1. **Designed to degrade *DMPK***
   Degrade *DMPK* mRNA to release sequestered splice proteins
2. **Designed to maintain *DMPK***
   Engage and open hairpin resulting in steric displacement of sequestered splice proteins

Designed to maintain *DMPK*

Our anti-gene opens the double-stranded RNA target to correct the splicing defect and is designed to maintain *DMPK* protein which is likely important in the muscle and brain of adults

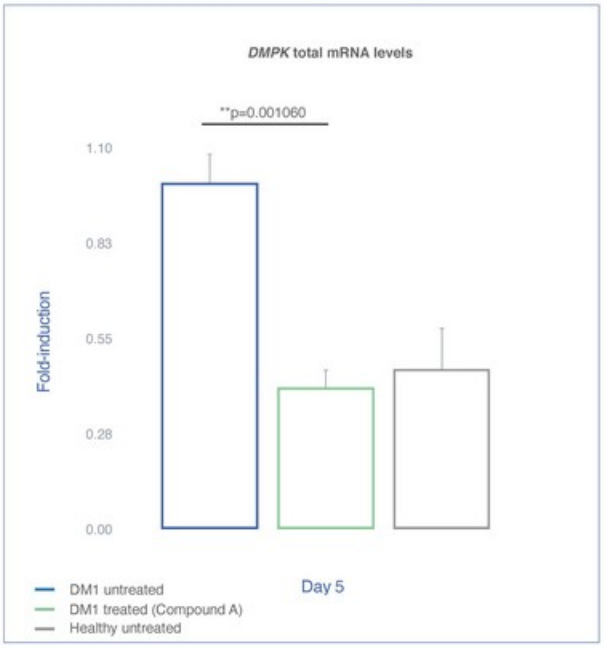
DM1 is a multi-system disease and we believe we provide a multi-system solution



In vitro activity

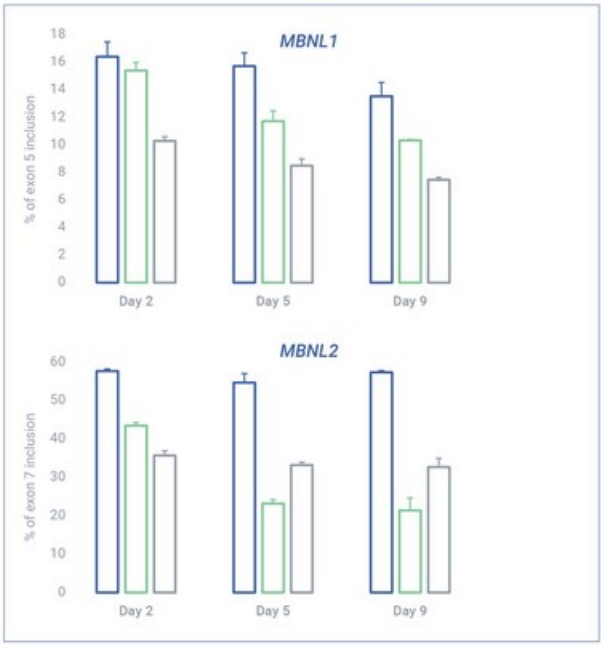
Target engagement in patient cells

The DM1 anti-gene (Compound A) traffics into the nucleus and engages and normalizes *DMPK* mRNA



Correction of mis-splicing in patient cells

Compound A rescues appropriate splicing across two established mis-spliced transcripts (*MBNL1* and *MBNL2*) with induction by day 2 which continues to mature over time

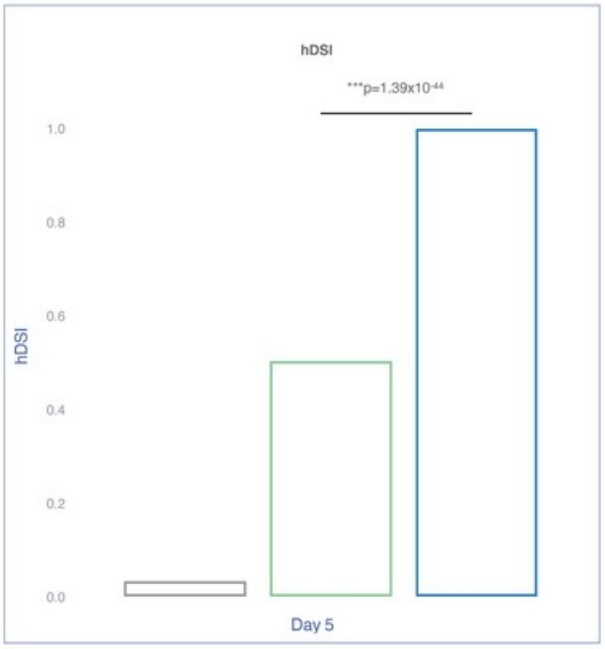


DM1 untreated
DM1 treated (Compound A)
Healthy untreated

Correction of mis-splicing in patient cells

Composite score of global splice rescue after administration of Compound A illustrates statistically significant broad correction across many transcripts (hDSI) at day 5

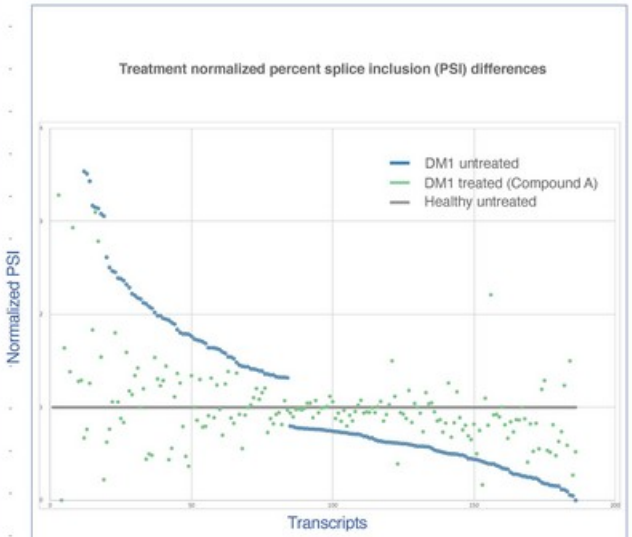
Human differential splice inclusion (hDSI) is calculated by first selecting a set of cassette exons with a) delta-percent splice inclusion (PSI) between healthy and untreated mice, as calculated by rMATS, of >= 20%, b) p-value of <= 0.05 for differential splicing as calculated by rMATS, and c) read depth >= 5x in all healthy, untreated and treated samples after polyA selected 2x150 RNASeq to >50m read depth. For each cassette exon a scale is calibrated from 0-1 where 0 is the mean PSI of healthy untreated samples and 1 is the mean PSI of DM1 untreated samples. For each sample the PSI for each cassette exon is normalized into the scale, and then values for all exons are averaged to yield hDSI.



DM1 untreated
DM1 treated (Compound A)
Healthy untreated

Correction of mis-splicing in patient cells

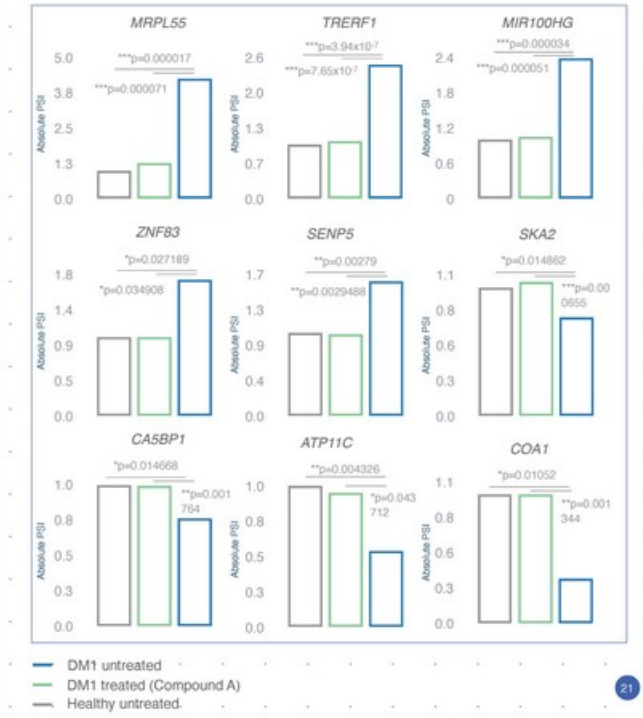
Statistically significant rescue of splicing is observed after Compound A treatment across >175 significantly mis-spliced human transcripts by day 5



Statistically significant (p<0.05) normalized percent splice inclusion (PSI) between DM1 treated with Compound A vs. DM1 untreated human cells is illustrated for >175 unique human transcripts (x-axis). Transcripts were pre-selected for >=20% PSI, >=5x fold-coverage after RNASeq and p<0.05 between healthy untreated and DM1 untreated PSI.

Correction of mis-splicing in patient cells

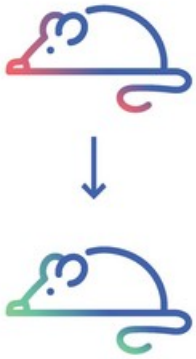
Restoration of normal splicing is observed across many transcripts by day 5 after treatment with Compound A



In vivo activity (single-dose IV)

The HSA^{LR} model[1]

[1]Mankodi A, Logigian E, Callahan L, McClain C, White R, Henderson D, Krym M, Thornton CA. Myotonic dystrophy in transgenic mice expressing an expanded CUG repeat. Science. 2000 Sep 8;289(5485):1769-73.



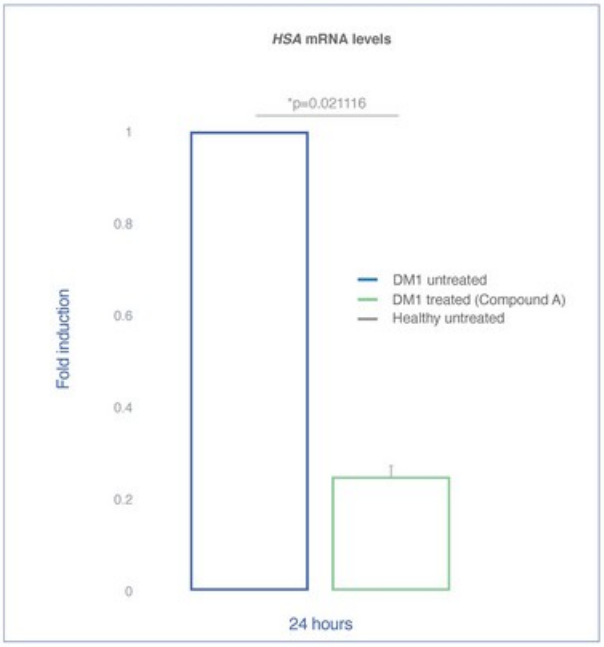
Splice aberrations in skeletal muscle leading to DM1 phenotype

IV DM1 anti-gene (Compound A) administration

Rapid induction of global splice rescue

Target engagement *in vivo*

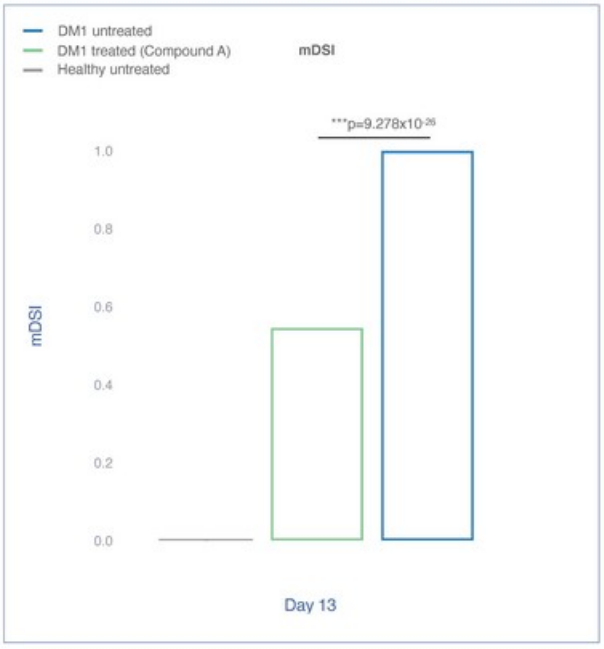
29 mg/kg single-dose injection of Compound A into the tail vein traffics to nuclei and engages target *HSA* (skeletal muscle actin) mRNA within 24 hours in Tibialis anterior skeletal muscle



Correction of mis-splicing in muscle

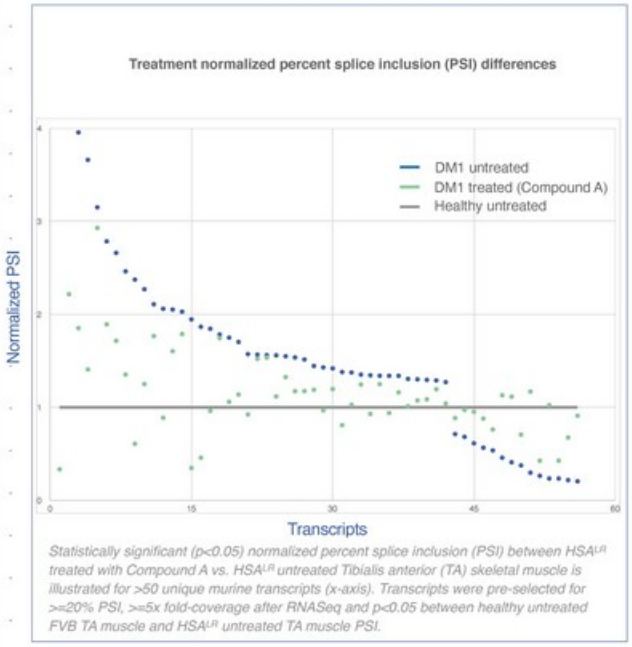
Composite score of global splice rescue after administration of Compound A as measured by RNA sequencing illustrates significant broad correction across many transcripts (mDSI) in Tibialis anterior skeletal muscle at day 13

Mouse differential splice inclusion (mDSI) is calculated by first selecting a set of cassette exons with a) delta-PSI between healthy untreated FVB strain TA muscle and untreated HSA^{LR} mouse TA muscle, as calculated by rMATS, of >= 20%, b) p-value of <= 0.05 for differential splicing as calculated by rMATS, and c) read depth >= 5x in all healthy untreated, DM1 untreated and DM1 treated samples after polyA selected 2x150 RNASeq to >50m read depth. For each cassette exon a scale is calibrated from 0-1 where 0 is the mean PSI of healthy untreated samples and 1 is the mean PSI of DM1 untreated samples. For each sample the PSI for each cassette exon is normalized into the scale, and then values for all exons are averaged to yield mDSI.



Correction of mis-splicing in muscle

Compound A treatment induces significant splice rescue across >50 Tibialis anterior skeletal muscle transcripts by day 13

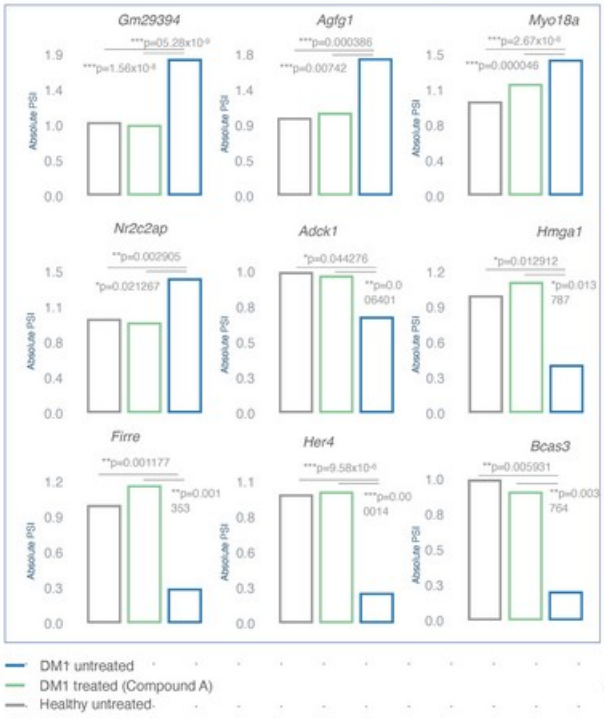


Statistically significant (p<0.05) normalized percent splice inclusion (PSI) between HSA^{LR} treated with Compound A vs. HSA^{LR} untreated Tibialis anterior (TA) skeletal muscle is illustrated for >50 unique murine transcripts (x-axis). Transcripts were pre-selected for >=20% PSI, >=5x fold-coverage after RNASeq and p<0.05 between healthy untreated FVB TA muscle and HSA^{LR} untreated TA muscle PSI.

Rapid and broad resolution of global mis-splicing

Restoration of normal splicing is observed across many transcripts in Tibialis anterior muscle by day 13 after intravenous administration

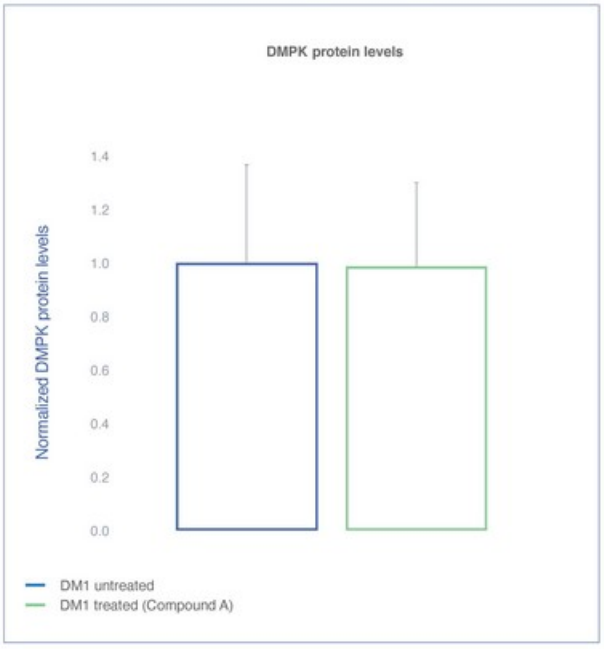
Compound A is well tolerated at the dose demonstrating activity *in vivo*



DMPK protein levels

Retention of DMPK protein

DMPK protein levels remain unchanged 5 days after initial dosing with Compound A in patient cells



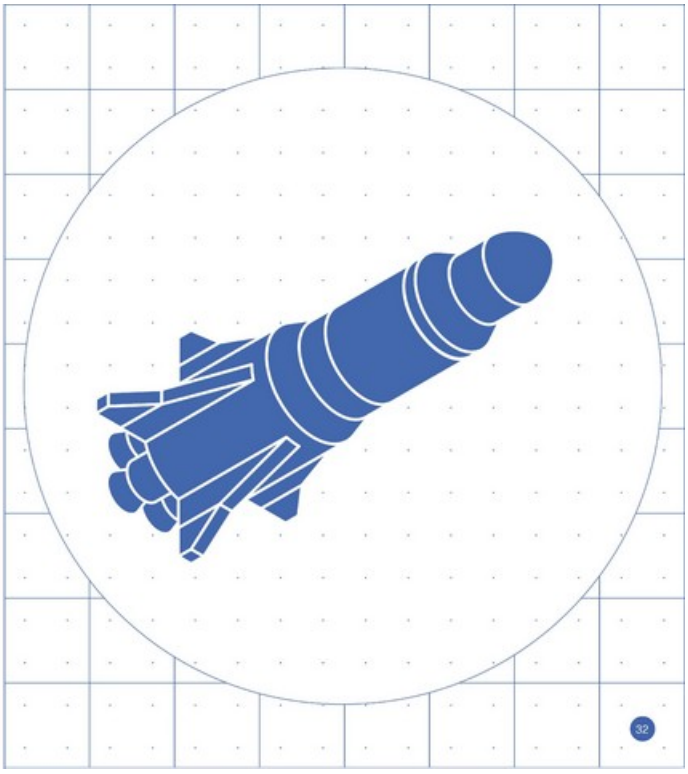
PATrOL™ technology platform

Unique ability to target any misbehaving nucleic acid

A modular system comprised of delivery technologies, scaffolds and nucleobases

Delivery technologies

Delivery modules allow broad or focused cell-type localization and enhancer modules increase potency



Delivery technologies

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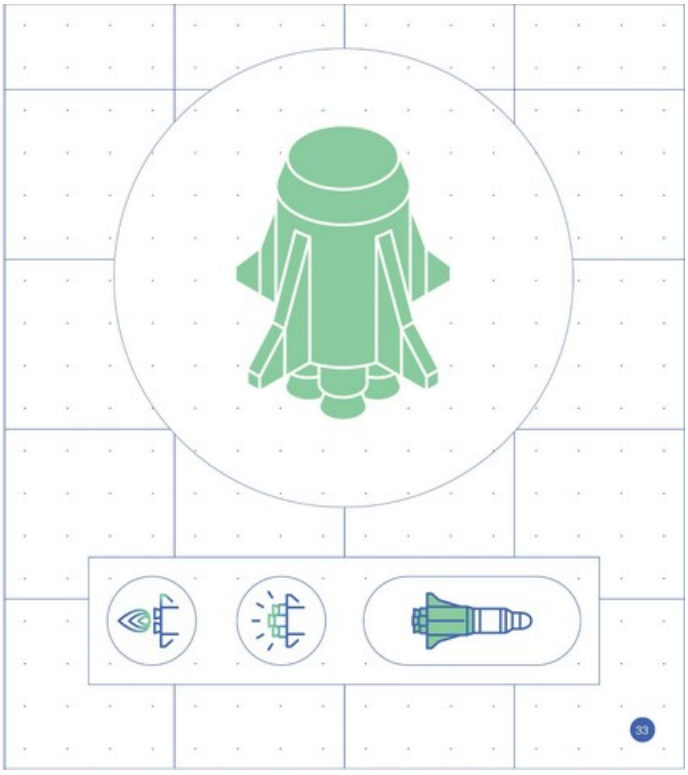

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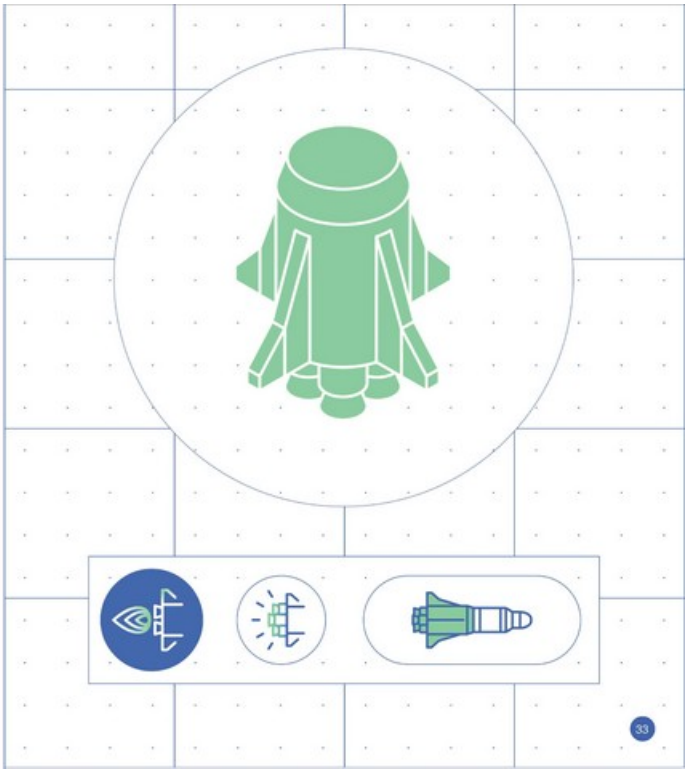
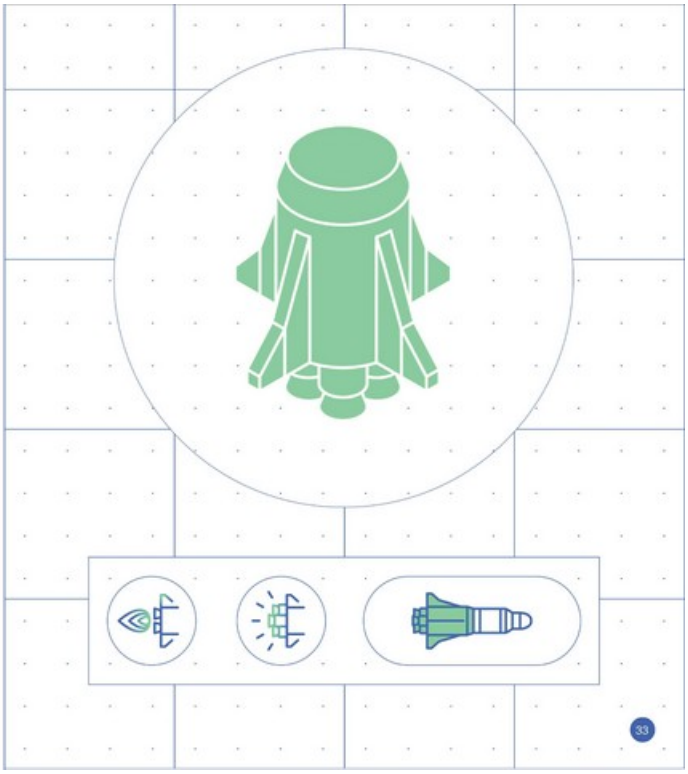


Synthetic poly-amide scaffolds

Ultra-precise target neutralization due to semi-rigidity, high binding affinity and neutral charge without the limitations of ribose backbones



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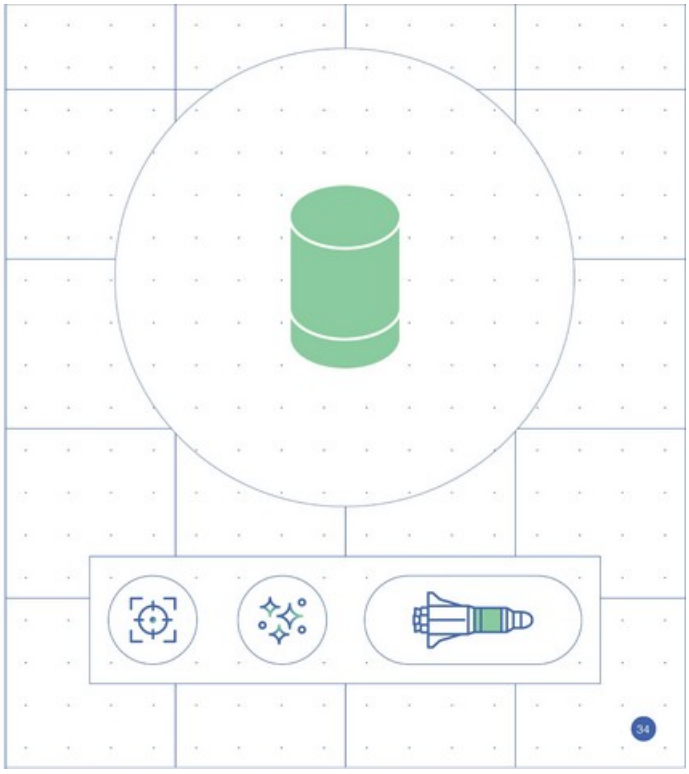

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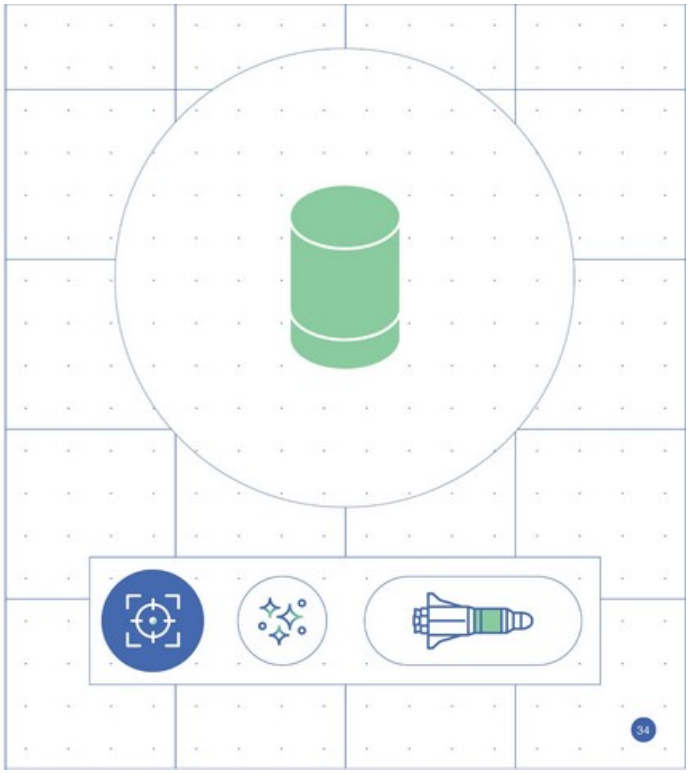
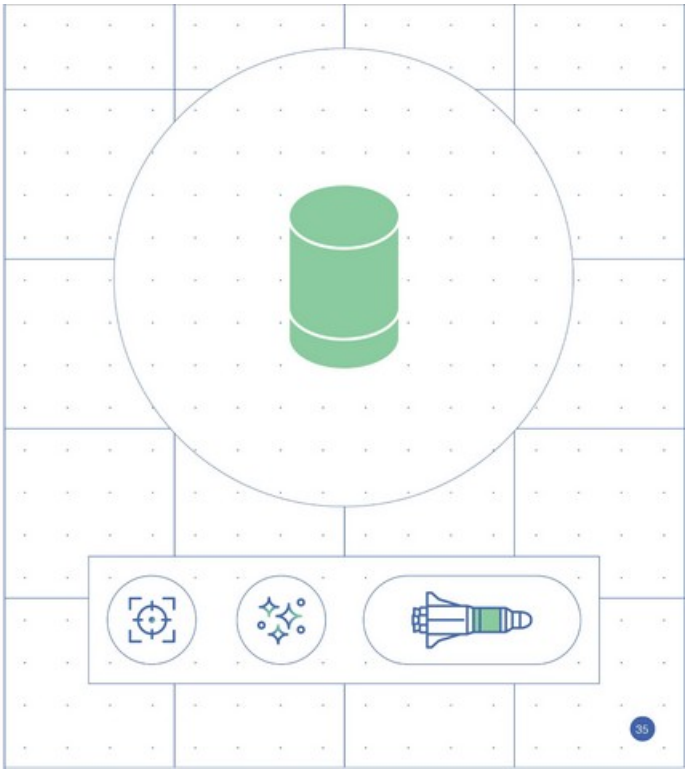


Nucleobases

Targeting with natural nucleobases and a portfolio of reimagined nucleobases to further improve specificity of target engagement



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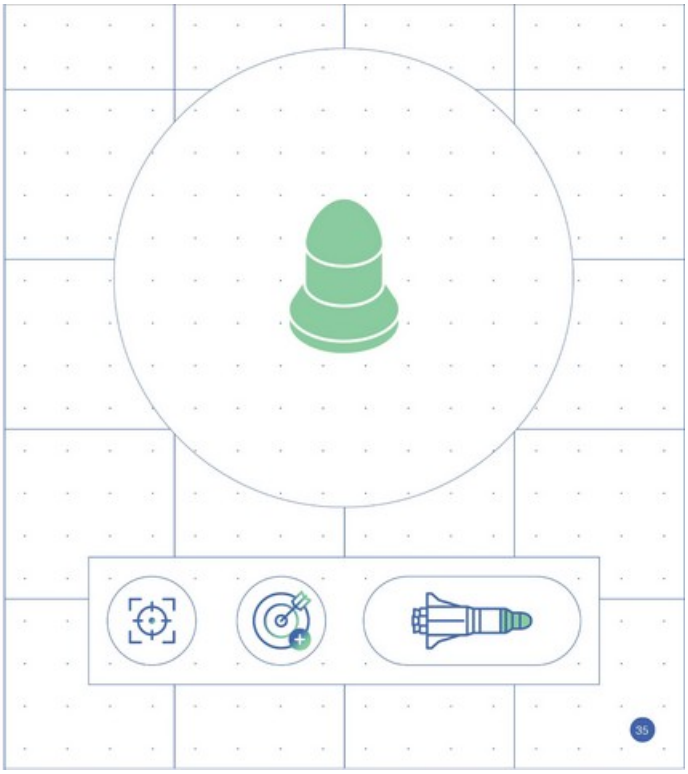

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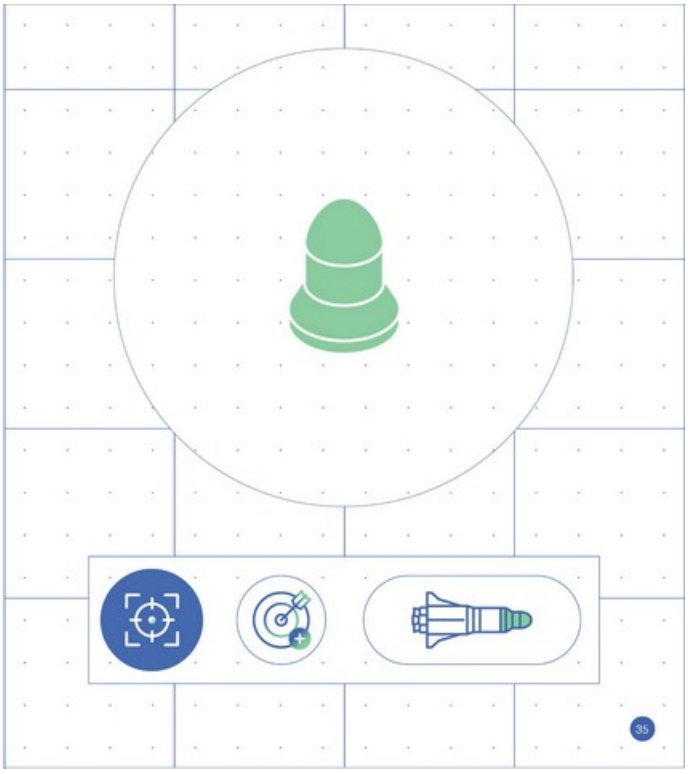
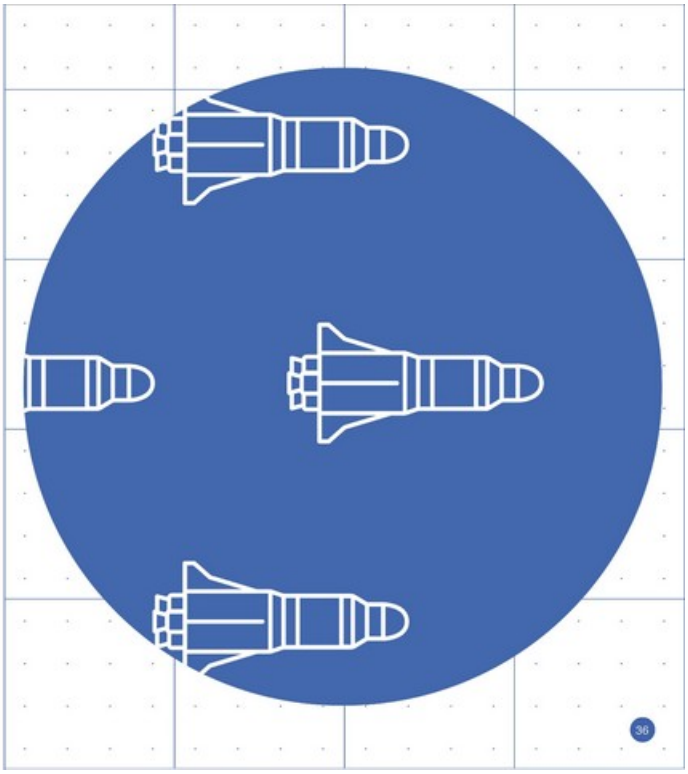


Our capabilities are unique

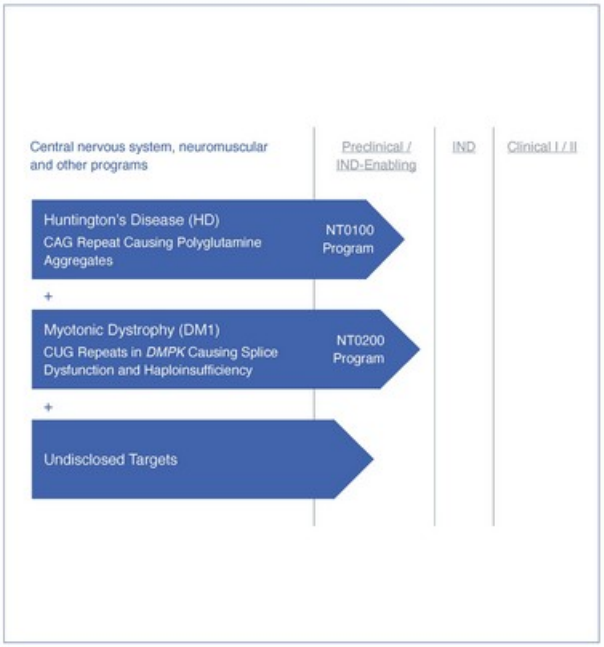
A platform enabling precision engagement with any genetic targets to increase, decrease or change resultant protein function and resolve disease



Pipeline

Emerging pipeline

A rigorous foundation will allow us to accelerate into many programs over time



The team

Experts in the technology and drug development

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AUDIT & COMPENSATION

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AUDIT & GOVERNANCE/ NOMINATING COMMITTEES

CINRYZE (C1 esterase inhibitor (human)) · CYTOGAM (Cytomegalovirus Immune Globulin Intravenous (Human)) (CMV-IVIG) · Dalvance (dalbavancin) for injection · Eraxis (anidulafungin IV) · Ethyol (amifostine) · INTELENCE (etravirine) · PREZISTA (darunavir) tablets · SYNAGIS (PALIVIZUMAB)

Summary

Take-aways

PATrOL: a validated disruptive technology

- *In vivo* data demonstrating target engagement and therapeutic activity after single IV administration
- Broad biodistribution demonstrated in NHPs
- Potential to increase, decrease, or change protein function in a single unified platform

Large markets with high unmet medical need

- Myotonic dystrophy, type 1 (DM1)
- Huntington's disease (HD)

Proven team of drug developers

Myotonic dystrophy, type 1

- A severe intractable genetic disease
- Affects >5/100,000 with large unmet need

Our data describing a solution to this disease

- Rapid induction of rescue across a multitude of mis-spliced transcripts *in vitro* and *in vivo*
- Novel delivery technology allows single-dose IV administration without conjugation to biologics
- Designed to not reduce DMPK protein
- Well tolerated at therapeutically active dose

Exhibit 99.2

NeuBase Therapeutics Announces Positive Preclinical *In Vivo* Data for PATrOL™-enabled Anti-gene for the Treatment of Myotonic Dystrophy Type 1

In vivo *data after single-dose IV administration demonstrate engagement with* DMPK *mRNA and broad rescue of mis-splicing across key transcripts.*

Findings provide support for hypothesized mechanism of action of anti-gene, which is designed to not degrade the DMPK *transcript.*

Data further validate the potential of the PATrOL™ platform to develop highly targeted therapies that increase, decrease or change causal protein function.

NeuBase management to hold conference call and webcast today, December 16, at 8:00 a.m. EST

PITTSBURGH, PA – December 16, 2020 – NeuBase Therapeutics, Inc. (Nasdaq: NBSE) ("NeuBase" or the "Company"), a biotechnology company accelerating the genetic revolution using a new class of synthetic medicines, today announced positive *in vitro* and *in vivo* preclinical data for its PATrOL™-enabled anti-gene therapies for the treatment of myotonic dystrophy type 1 (DM1). These new data show that PATrOL-enabled Compound A can rapidly resolve mis-splicing without negatively impacting DMPK protein levels. They also support the potential of NeuBase's anti-gene approach to comprehensively treat the underlying cause of DM1.

"Despite the fact that the genetic basis of DM1 is well understood today, there is still an urgent need to find the first genetically-targeted, disease-modifying treatment option for affected patients," said Curt Bradshaw, Ph.D., Chief Scientific Officer of NeuBase. "DM1 is caused by a genetic mutation in the *DMPK* gene leading to mis-splicing of a broad spectrum of genes and DMPK protein insufficiency. A treatment option that addresses mis-splicing while retaining functional DMPK protein levels may be key to treating all aspects of DM1."

Dietrich A. Stephan, Ph.D., Chief Executive Officer of NeuBase, added, "Using our proprietary PATrOL platform, we have designed a first-in-class anti-gene candidate that selectively binds mutant *DMPK* mRNA and opens its hairpin secondary structure, as opposed to a mechanism of action that explicitly degrades the mutant and wild-type transcripts indiscriminately, making it a unique option for the treatment of DM1. These *in vitro* and *in vivo* data both support our hypothesized mechanism of action and demonstrate rapid and broad resolution of the mis-splicing that is the primary cause of DM1.

"This is the second set of positive data that we've announced in 2020 for our PATrOL-enabled therapies, which we believe serves as proof of concept that further validates our technologic foundation. With a single unified platform, we believe we can increase, decrease or change protein function of potentially any nucleic acid target, unique among genetic medicine approaches. We are excited by the progress we have made and look forward to providing additional updates on our platform and pipeline of programs at an R&D day in the first half of 2021."

In vitro data highlights in DM1 patient-derived fibroblasts:

- Compound A traffics to the nucleus, engages and normalizes *DMPK* mRNA.
- Compound A rescues mis-splicing of two key DM1 dysregulated transcripts (*MBNL1* and *MBNL2)* within two days after initial treatment. Notably, induction of rescue continues to improve through day 9, the latest time point analyzed.
- Compound A significantly induces broad correction of global exon inclusion levels of mis-spliced transcripts.
    - Statistically significant improvement in global splicing as measured by the human differential splice inclusion (hDSI) statistic.
    - More than 175 dysregulated human transcripts achieved statistically significant improvement in splicing, many with completely normalized exon usage.
- DMPK protein levels remain unchanged 5 days after a single Compound A dose, supporting the hypothesized mechanism of action maintaining DMPK.

In vivo data highlights in the HSA^{LR} transgenic mouse model of DM1 that expresses high levels of mutant CUG-repeat-containing mRNA (*HSA*) in skeletal muscle:

- A single intravenous (IV) injection of 29 mg/kg of Compound A traffics to the nucleus and engages *HSA* mRNA within 24 hours in tibialis anterior (TA) skeletal muscle.
- A single intravenous (IV) injection of Compound A significantly induces broad correction of global exon inclusion levels of mis-spliced transcripts in HSA^{LR} TA skeletal muscle at day 13.
- Statistically significant improvement in global splicing as measured by the murine differential splice inclusion (mDSI) statistic.
    - More than 50 unique dysregulated murine transcripts achieved statistically significant improvement in splicing post-treatment, with many achieving complete normalization of appropriate exon usage.
- Compound A was well tolerated after single dose administration at the dose demonstrating activity *in vivo.*

DM1 is a rare, autosomal dominant repeat expansion disorder characterized by progressive muscle wasting and weakness. It also affects the central nervous system (CNS) and heart. DM1 is caused by CTG nucleic acid repeats in the *DMPK* gene that produce a hairpin structure in the transcribed *DMPK* mRNA. The hairpin structure sequesters critical splice regulators and results in the mis-splicing of multiple gene transcripts. Furthermore, the binding of splice regulators traps the mutant *DMPK* mRNA in the nucleus, resulting in DMPK protein haploinsufficiency, or half the level of protein that is needed for normal function, which is thought to exacerbate the CNS and cardiac symptoms that are characteristic of DM1 (as knock-out mice for *Dmpk* show both severe cardiac conduction defects as well as issues with neuronal cytoskeletal remodeling manifesting in aberrant long-term potentiation). The prevalence of DM1 is >5/100,000 in the general population. There are currently no approved treatments for DM1.

Conference Call and Webcast Details

NeuBase Therapeutics, Inc. will discuss these data during a webcasted conference call with slides today, December 16, 2020, at 8:00 a.m. EST. To access the webcast, please click here. An archived recording of this presentation will be available following the call through the IR Calendar page on the Investors section of the Company's website, www.neubasetherapeutics.com.

About NeuBase Therapeutics, Inc.

NeuBase is accelerating the genetic revolution using a new class of synthetic medicines. NeuBase's designer PATrOL™ therapies are centered around its proprietary drug scaffold to address genetic diseases at the source by combining the highly targeted approach of traditional genetic therapies with the broad organ distribution capabilities of small molecules. With an initial focus on silencing disease-causing mutations in debilitating neurological, neuromuscular and oncologic disorders, NeuBase is committed to redefining medicine for the millions of patients with both common and rare conditions. 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 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 significance and implications of the Company's positive *in vitro* and *in vivo* preclinical data for its PATrOL™-enabled anti-gene therapies for the treatment of myotonic dystrophy. 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, 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 develop and commercialize its product candidates; the timing of initiation of the Company's planned clinical trials; the risks that prior data will not be replicated in future studies; 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 future product candidates; the clinical utility, potential benefits and market acceptance of the Company's 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 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.

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