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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.

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.
NeuBase Therapeutics
Next-Generation Peptide Nucleic Acids (“PNAs”) Addressing Rare Genetic Disease

**First-in-class technology**
- PATrOL™ platform capable of addressing a wide range of genetic diseases and cancers
- Advantages of small molecules combined with selectivity of antisense oligonucleotides (ASOs)
- Strong IP composition of matter & field of use through 2037+

**Key advantages over traditional antisense oligonucleotides**
- PATrOL™ platform enables rapid drug design, blood brain barrier penetration, broad systemic distribution for multi-tissue disease, increased cell permeability, and accessing of genomic loci and secondary RNA structures

**Initial focus on rare repeat expansion disorders**
- High unmet need, orphan focus
- Ongoing development for Huntington’s disease (NT0100) and myotonic dystrophy (NT0200), with billion dollar peak sales opportunity in each indication

**Ability to expand into additional indications**
- Modular design lends ability to address a wide range of rare, genetic diseases
- Approach can be utilized for DNA therapeutic techniques, such as gene editing and gene regulation, as well as liquid biopsy testing
Board of Directors

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VIVIDION Therapeutics

MAYO CLINIC

Lilly

AISLING CAPITAL

LOXO ONCOLOGY

MedImmune

PharmAthene
Global Unmet Need: Genetic Disease

5,000-7,000
Individual Rare Diseases

Collectively Account for up to 10% of Global Population

95%
Have No Effective Therapeutics

COUNTLESS Orphan Indications
After DNA is transcribed into RNA, a process known as splicing, which removes intervening sequences to form messenger RNA (mRNA), occurs.

That mRNA is then translated into a protein.
The PATrOL™ Platform: Dominant and Recessive Disease

Mutated DNA → TRANSCRIPTION → Mutant RNA

SYSTEMIC ADMINISTRATION

INHIBIT TRANSLATION
ALTER SPLICING

TRANSLATION → Disease-Causing Protein

MODULATE DISEASE
Spinraza (Biogen) is an antisense oligonucleotide for Spinal Muscular Atrophy that was approved in late 2016.

Set to be one of the best launches for a rare disease drug ever.

Developed by Ionis Pharmaceuticals with Biogen.

Limitations to the technology result in unfavorable intrathecal (spinal cord) route of administration.

NeuBase’s two initial indications have larger global prevalence numbers than SMA (HD=5.7/100,000 and DM1=5/100,000 vs. SMA=1-2/100,000)\(^2\).

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Advantages of PATrOL™ over Traditional ASOs

<table>
<thead>
<tr>
<th>COMPOUND PROPERTY</th>
<th>ASOS</th>
<th>NEUBASE</th>
<th>NEUBASE ADVANTAGE</th>
</tr>
</thead>
<tbody>
<tr>
<td>Modular molecular design</td>
<td>✗</td>
<td>✓</td>
<td>Platform enables many applications</td>
</tr>
<tr>
<td>Proprietary engineered nucleobases</td>
<td>✗</td>
<td>✓</td>
<td>Tuning to minimize off-target effects</td>
</tr>
<tr>
<td>Favorable length (3-mer+)</td>
<td>✗</td>
<td>✓</td>
<td>Compounds better reach target tissue</td>
</tr>
<tr>
<td>Ability to open up and bind to double-stranded RNA</td>
<td>✗</td>
<td>✓</td>
<td>Higher specificity to mutant allele, more effective deactivation of disease-related mRNA</td>
</tr>
<tr>
<td>No self-aggregation</td>
<td>✗</td>
<td>✓</td>
<td>No toxic aggregation of drug</td>
</tr>
<tr>
<td>Innately stable to enzymes</td>
<td>✗</td>
<td>✓</td>
<td>Stable in circulation; resistant to premature degradation</td>
</tr>
<tr>
<td>Endosome-independent cell permeability</td>
<td>✗</td>
<td>✓</td>
<td>Directly reach intracellular target in cytoplasm and nucleus</td>
</tr>
<tr>
<td>Broad tissue distribution</td>
<td>✗</td>
<td>✓</td>
<td>Address all tissue pathologies; even distribution across all tissue types</td>
</tr>
<tr>
<td>Ability to cross blood brain barrier (BBB)</td>
<td>✗</td>
<td>✓</td>
<td>Favorable systemic route of administration</td>
</tr>
<tr>
<td>Capable of gene silencing and gene splicing</td>
<td>✗</td>
<td>✓</td>
<td>Potential to address dominant and recessive genetic diseases</td>
</tr>
<tr>
<td>Also targets DNA</td>
<td>✗</td>
<td>✓</td>
<td>Future opportunities in gene editing and regulation</td>
</tr>
</tbody>
</table>
The PATrOL™ Platform

<table>
<thead>
<tr>
<th>Peptide Backbone</th>
<th>Engineered Nucleotides</th>
<th>Cell / BBB Penetration Moiety</th>
<th>Self-assembly at the Target RNA</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Protease and nuclease resistant</td>
<td>• Increase selectivity to mutant transcript by tuning H-bonding</td>
<td>• Enables “active translocation” across cell membranes</td>
<td>• Moieties added to ends of oligos to enable end-to-end linking across target</td>
</tr>
<tr>
<td>• Pre-organized with no “stereoisomers”</td>
<td>• Access and bind secondary and tertiary structures with bi-facial bases</td>
<td>• Broad and uniform tissue distribution</td>
<td>• Allows short oligos to become selective when linked together</td>
</tr>
<tr>
<td>• Not immunogenic (TLR, innate, adoptive)</td>
<td>• Enables smaller drugs that act like small molecules</td>
<td>• Delivers ASO across the BBB after systemic administration</td>
<td>• Provides selectivity to expanded trinucleotide repeat structures</td>
</tr>
<tr>
<td>• Non-polar for tighter binding</td>
<td></td>
<td>• Long residence time within cells</td>
<td></td>
</tr>
<tr>
<td>• Acts through steric hindrance of splicing, translation, etc.</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
In Vitro Target Engagement and Protein Knock-Down

Legend: PATrOL™ designed to target the EGFR transcript transfected into two cancer cell lines (head and neck, non-small cell lung) along with controls including a “sense” and scrambled PATrOLs™. Quantification measured (Left) by specific RT-PCR of the EGFR transcript and (Right) by immunoblotting with an anti-EGFR antibody.

In Vitro Dose Dependent Target Engagement and Cell Senescence

Legend: PATrOL™ designed to target the EGFR transcript transfected into two cancer cell lines (Left: head and neck, Right: non-small cell lung) along with controls including scrambled PATrOLs™ at increasing concentrations. Quantification measured ATP metabolism by luminescence.

In Vivo Biodistribution to Target Tissue, Cell Permeability and Residence Time on RNA Target of >4 Hours Post Systemic Injection (IP)

**Left.** PATrOL™ designed to target the EGFR transcript delivered through intraperitoneal (IP) injection into a UMSCC-U22 mouse xenograft (on the back) compared to **Right** non-targeted PATrOLs™. Red= EGFR PATrOL™, Blue=nuclei and Green=cytosolic actin.
In Vivo Results in Tumor Efficacy Comparable to Two FDA-Approved Drugs Addressing the Same Target in Xenograft Models

**Legend:** PATrOL™ designed to target the EGFR transcript through intraperitoneal (IP) injection into a UMSCC-U22 mouse xenograft (on the back) compared to modified scrambled PATrOLs™ and two FDA-approved drugs that target EGFR protein. Tumor volume was measured to 17.5 days post-treatment.

## Development Pipeline

<table>
<thead>
<tr>
<th>Repeat Expansion</th>
<th>Preclinical / IND-enabling</th>
<th>IND</th>
<th>Clinical I/II</th>
</tr>
</thead>
<tbody>
<tr>
<td>Huntington’s Disease (HD) Polyglutamine CAG Repeats</td>
<td>NT0100</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Myotonic Dystrophy (DM1) CTG Repeats</td>
<td>NT0200</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Polyalanine Diseases CGN Repeats</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Dominant Genetic Disorders</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Familial Parkinson’s Disease LRRK2 &amp; SNCA</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Erythermalgia</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Oncogenic Mutations</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>hTERT</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
A potentially transformative therapy for Huntington’s disease (HD)
Huntington’s disease (HD) is a devastating rare neurodegenerative disorder, affecting 5.7 in 100,000 individuals in North America and Europe.

Caused by toxic aggregation of mutant huntingtin protein, leading to progressive neuron loss in the striatum and cortex of the brain.

There is no approved therapy that has been shown to delay or halt disease progression.

Due to the importance of HTT for normal development and function, it is key for an HD therapy to limit the production of the mutant protein while leaving the activity of the wildtype protein intact.
Systemic Administration Penetrates Deep Brain Structures

NT-0100.A
5mg/kg IP single dose injection
Image brain after 8 hours

Choroid Plexus:
Blood-CSF Barrier

Caudate Putamen:
Site of HD Pathology
Broad Tissue Uptake after Systemic Injection

Single-faced 6-mer PATrOL™ molecules were incubated with a 12 repeat synthetic expansion mimicking a mutant allele of the Huntington’s disease gene.

A melting curve experiment shows dissociation of the concatenated PATrOL™ at a temperature that far exceeds the melting point of 6-mers.
After transcription, RNA binds itself and forms stable secondary structures. Traditional ASOs are not capable of accessing secondary RNA structures such as hairpins and loops.

PATrOL-enabled therapies are designed with a modified peptide backbone and engineered nucleobases, uniquely allowing NeuBase’s therapies to “wedge” into double-stranded target RNA in its native secondary structure.

Accessing secondary structures allows for higher specificity to the mutant allele and for more effective deactivation of disease-related RNA before it can be translated into damaging protein.
NT0200
Disrupting the myotonic dystrophy disease pathway
Myotonic Dystrophy 1 (DM1) is a highly degenerative muscular condition affecting 1 in every 20,000 people around the world. The condition is marked by muscle fatigue affecting different regions of the body, such as hands, face, neck and lower legs as well as multi-system pathology.

DM1 is caused by a mutated DMPK gene, which produces an expanded version of DMPK mRNA and then aggregates inside cells due to its abnormal length. These aggregates interfere with cellular function.

It is key for a DM1 therapy to access the secondary structure created by the CUG repeats in order to specifically target the mutant allele.
PATrOL™ Platform: Bifacial Janus Bases

NeuBase is the first and only company to successfully create bifacial Janus bases, engineered nucleic acids that target double-stranded DNA or RNA by engaging both strands at once.

(A) is a 3-mer Janus PNA with concatenation linkers on the 5’ and 3’ ends. (B) The molecular reaction that results in bifacial binding to the repeat expansion. These Janus PATrOLs™ can also self-deactivate if they do not find their target.

PATrOL™ technology is capable of discriminating the mutant from the wild type DMPK allele

PATrOL™ technology disrupts the CUG-MBNL1 complex, demonstrating ability to interfere with DM1 disease pathway

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Wei-Che Hsieh; Raman Bahal; Shivaji A. Thadke; Kirti Bhatt; Krzysztof Sobczak; Charles Thornton; Danith H. Ly. Biochemistry 2018, 57, 907-911. DOI: 10.1021/acs.biochem.7b01239
PATrOL™ Beyond HD and DM1
NT0100 and NT0200 Beyond HD and DM1

NT0100 could potentially be applicable in all 8 CAG (poly-glutamine) repeat diseases

NT0200 could potentially be applicable in three indications

NeuBase Therapeutics holds an exclusive license to the PATrOL™ technology, with 9 patents and applications covering matter and field of use of the platform.

Patents have 2037 expiration, not including “Hatch Waxman” extensions.

USPTO has allowed claims for Composition of Matter.

Developed at Carnegie Mellon University.
## Examples of Antisense Out-Licensing Transactions

Select Companies Targeting RNA with Traditional ASOs

<table>
<thead>
<tr>
<th>Date</th>
<th>Company</th>
<th>Deal</th>
<th>Indication</th>
<th>Clinical Phase</th>
<th>Value</th>
<th>Partner</th>
</tr>
</thead>
<tbody>
<tr>
<td>2018</td>
<td>Ionis Pharmaceuticals</td>
<td>Development, License, Commercialization</td>
<td>Several Indications</td>
<td>-</td>
<td>$1b</td>
<td>Biogen</td>
</tr>
<tr>
<td>2018</td>
<td>Ionis Pharmaceuticals</td>
<td>License agreement</td>
<td>Kidney Disease</td>
<td>Undisclosed</td>
<td>$30m upfront, $300m milestones</td>
<td>AstraZeneca</td>
</tr>
<tr>
<td>2018</td>
<td>Wave Life Sciences</td>
<td>Development &amp; Commercialization (3 candidates)</td>
<td>Neurological Disorders</td>
<td>1b/2a</td>
<td>$110m upfront, $60m research</td>
<td>Takeda</td>
</tr>
<tr>
<td>2017</td>
<td>Ionis Pharmaceuticals</td>
<td>License agreements (2 candidates)</td>
<td>GI Autoimmune</td>
<td>Undisclosed</td>
<td>$5m &amp; $10m, $800m milestones</td>
<td>Janssen</td>
</tr>
<tr>
<td>2017</td>
<td>Ionis Pharmaceuticals</td>
<td>License agreement</td>
<td>Huntington’s Disease</td>
<td>1/2a</td>
<td>$100m upfront, $363m milestones</td>
<td>Roche</td>
</tr>
<tr>
<td>2017</td>
<td>Akcea Therapeutics &amp; Ionis</td>
<td>License agreement (2 candidates)</td>
<td>Cardiovascular Disease</td>
<td>2</td>
<td>$75m, $1b milestones</td>
<td>Novartis</td>
</tr>
<tr>
<td>2016</td>
<td>Ionis Pharmaceuticals</td>
<td>License agreement</td>
<td>Spinal Muscular Atrophy</td>
<td>2</td>
<td>$75m upfront, $150m milestones</td>
<td>Biogen</td>
</tr>
<tr>
<td>2015</td>
<td>Isis (now Ionis) Pharmaceuticals</td>
<td>License agreement</td>
<td>Kidney Disease</td>
<td>2</td>
<td>$100m upfront, $375m milestones</td>
<td>Bayer</td>
</tr>
</tbody>
</table>
NeuBase Therapeutics

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