# 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 10, 2019**

# WAVE LIFE SCIENCES LTD.
**(Exact name of registrant as specified in its charter)**

| **Singapore** | **001-37627** | **Not Applicable** |
|---|---|---|
| **(State or other jurisdiction of incorporation)** | **(Commission File Number)** | **(IRS Employer Identification No.)** |

| | |
|---|---|
| **7 Straits View #12-00, Marina One East Tower Singapore 018936** | **018936** |
| **(Address of principal executive offices)** | **(Zip Code)** |

**Registrant's telephone number, including area code: +65 6236 3388**

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 (see General Instruction A.2. below):

☐ 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))

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

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. ☐

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

| Title of each class | Trading symbol(s) | Name of each exchange on which registered |
|---|---|---|
| **$0 Par Value Ordinary Shares** | **WVE** | **The Nasdaq Global Market** |

**Item 2.02 Results of Operations and Financial Condition.**

On May 10, 2019, Wave Life Sciences Ltd. (the "Company") announced its financial results for the quarter ended March 31, 2019. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated by reference herein.

**Item 7.01 Regulation FD Disclosure.**

From time to time, the Company presents and/or distributes slides and presentations to the investment community to provide updates and summaries of its business. On May 10, 2019, the Company updated its corporate presentation, which is available on the "For Investors & Media" section of the Company's website at http://ir.wavelifesciences.com/. This presentation is also furnished as Exhibit 99.2 to this Current Report on Form 8-K.

*The information in these Items 2.02 and 7.01 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, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.*

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

The following exhibits relating to Items 2.02 and 7.01 are furnished and not filed:

| Exhibit No. | Description |
|---|---|
| 99.1 | Press Release issued by Wave Life Sciences Ltd. dated May 10, 2019 |
| 99.2 | Corporate Presentation of Wave Life Sciences Ltd. dated May 10, 2019 |

**SIGNATURES**

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.

**WAVE LIFE SCIENCES LTD.**

By: */s/ Keith C. Regnanate*
Keith C. Regnante
Chief Financial Officer

Date: May 10, 2019

**Wave Life Sciences Reports First Quarter 2019 Financial Results and Provides Business Update**

**CAMBRIDGE, Mass., May 10, 2019** – Wave Life Sciences Ltd. (Nasdaq: WVE), a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases, today announced financial results for the first quarter ended March 31, 2019 and provided a business update.

“Since the start of the year, we have made significant progress advancing suvodirsen, our lead clinical program for the treatment of Duchenne muscular dystrophy patients amenable to exon 51 skipping, and we look forward to providing dystrophin biopsy data in the second half of the year. In parallel, initial commercialization activities are underway to support the potential approval and launch of this investigational therapy, first in the United States and then globally,” said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. “Suvodirsen is the first of multiple development programs we intend to advance for Duchenne, potentially enabling us to reach more patients living with DMD. Beyond our Duchenne programs, we are advancing our differentiated PRECISION-HD clinical program, an allele-selective approach to treating Huntington’s disease, and we look forward to reporting topline clinical results from these HD studies by year-end.”

**Business Update**

Wave is committed to building a fully integrated genetic medicines company led by its clinical- and preclinical-stage programs for the treatment of neuromuscular, central nervous system and ophthalmologic diseases.

**Neuromuscular Diseases**

**Initial commercialization activities ongoing following completion of suvodirsen Phase 1 clinical trial; efficacy data from ongoing open-label extension study expected this year**

- In April 2019, Wave announced the final results from its Phase 1 clinical trial of investigational suvodirsen (WVE-210201) in boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping. The results demonstrated a favorable safety and tolerability profile of suvodirsen for continued clinical development in the ongoing open-label extension (OLE) study and planned Phase 2/3 clinical trial.
- Suvodirsen is currently being studied in an OLE study, initiated in August 2018 with patients from the Phase 1 clinical trial. Wave expects to deliver an interim analysis of dystrophin expression from muscle biopsies in boys receiving suvodirsen in this study in the second half of 2019.
- The company expects to file for an accelerated approval of suvodirsen in the United States in the second half of 2020, pending positive clinical dystrophin expression data.

**DYSTANCE 51, Wave's Phase 2/3 clinical trial of suvodirsen in DMD, intended to support global regulatory filings**

- In April 2019, Wave announced the design of DYSTANCE 51, the planned Phase 2/3 efficacy and safety clinical trial of suvodirsen. The trial is designed to enroll boys who are between 5 and 12 years of age (inclusive) with a genetically confirmed diagnosis of DMD amenable to exon 51 skipping therapy. The DYSTANCE 51 primary efficacy endpoints will measure change in dystrophin protein level and change in the North Star Ambulatory Assessment score. In addition, the trial will include multiple functional outcome measures as secondary efficacy endpoints.
- In January 2019, the company announced that DYSTANCE 51 was selected for the U.S. Food and Drug Administration (FDA) complex innovative trial designs pilot program. Through this program, Wave intends to reduce the number of patients required to deliver conclusive clinical efficacy results, thereby minimizing the number of patients required in the placebo treatment arm and potentially accelerating study completion. Through participation in the program, the company has met with FDA staff to discuss design elements of the trial.
- DYSTANCE 51 is expected to be initiated in July 2019 and the company intends to use the results of this trial to seek regulatory approvals globally.

**Aiming to bring meaningful dystrophin protein restoration to more patients living with DMD**

- Wave is leveraging learnings from its ongoing DMD development and discovery efforts to advance WVE-N531, its preclinical candidate to treat DMD in boys amenable to exon 53 skipping. WVE-N531 induced up to 71% dystrophin protein restoration in DMD *in vitro* patient-derived myoblasts compared with healthy human myoblasts as measured by western blot. Subject to submission of clinical trial applications and approval to proceed, Wave expects to deliver topline clinical data for WVE-N531 in the second half of 2020.
- The company is also exploring exon targets beyond those targeted by suvodirsen and WVE-N531, including exons 44, 45, 52, 54 and 55.

**Central Nervous System (CNS) Diseases**

**Advancing PRECISION-HD clinical program, the first allele-selective approach for Huntington's disease patients**

- Wave's PRECISION-HD program, which consists of two global Phase 1b/2a clinical trials evaluating investigational therapies WVE-120101 and WVE-120102 for patients with Huntington's disease (HD), is continuing to enroll patients globally. The company expects to report topline clinical data from the PRECISION-HD program by the end of the year. These results are expected to include a summary of clinical safety results, the degree of mutant huntingtin protein lowering in cerebrospinal fluid (CSF) and the ratio of total huntingtin versus mutant huntingtin protein in CSF to assess wild-type huntingtin protein.
- WVE-120101 and WVE-120102, which selectively target the mutant allele of the *huntingtin* (*HTT*) gene, have been shown to reduce levels of mutant *HTT* mRNA and protein, while leaving wild-type or healthy *HTT* mRNA and protein largely intact in *in vitro* studies with patient-derived cell-lines. The healthy transcript is required to produce healthy HTT protein which is critical for neuronal function. Multiple preclinical studies in the literature indicate that long-term suppression of healthy HTT protein may have detrimental consequences. Wave's allele-specific approach may also enable the company to address the pre-manifest, or asymptomatic, HD patient population in the future.

**Leveraging PRISM to optimize C9ORF72 program and potential future CNS candidates**

- Wave announced today that it has further optimized its *C9ORF72*-targeting program in amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) and is advancing a new lead candidate (WVE-C092), which preferentially targets the transcript containing the GGGGCC (G4C2) expansion in the *C9ORF72* gene. The company leveraged advances in PRISM, its discovery and drug development platform, to design a candidate with an improved profile, including a substantial increase in potency in a preclinical study over its prior lead candidate. The observed potency and the expected durability of WVE-C092 may allow dosing frequency to be substantially optimized.

- Subject to the submission of clinical trial applications and approval to proceed, the company would expect to initiate clinical development of WVE-C092 in the second half of 2020.
- The company is utilizing the learnings from PRISM to design additional stereopure oligonucleotides with optimized profiles across other CNS diseases as part of its ongoing collaboration with Takeda.

**Ophthalmologic Diseases**

- Wave continues to advance stereopure oligonucleotides for the potential treatment of inherited retinal diseases. Preclinical data demonstrated that a single intravitreal injection of stereopure oligonucleotide in the eye of non-human primates resulted in greater than 95% knockdown of a target RNA in the retina for at least four months. Based on these data, the company is working to design clinical candidates that could achieve a therapeutic effect with only two doses per year. The company expects to announce its first ophthalmology candidate in the second half of 2019.

**First Quarter 2019 Financial Results and Financial Guidance**

Wave reported a net loss of $44.2 million in the first quarter of 2019 as compared to $35.2 million in the same period in 2018. The increase in net loss in the first quarter of 2019 was largely driven by increased research and development efforts and continued organizational growth to support Wave's corporate goals.

Research and development expenses were $40.1 million in the first quarter of 2019 as compared to $29.2 million in the same period in 2018. The increase in research and development expenses in the first quarter was primarily due to increased external expenses related to our suvodirsen clinical activities as well as increased investments in PRISM and other research and development expenses.

General and administrative expenses were $10.9 million in the first quarter of 2019 as compared to $8.0 million in the same period in 2018. The increase in general and administrative expenses in the first quarter was mainly driven by increases in employee headcount to support Wave's corporate goals, as well as increases in other general operating expenses.

As of March 31, 2019, Wave had $287.6 million in cash and cash equivalents as compared to $174.8 million as of December 31, 2018. The increase in cash and cash equivalents was mainly due to the $161.8 million in net proceeds from the January 2019 follow-on offering, partially offset by Wave's year-to-date net loss of $44.2 million.

Wave expects that its existing cash and cash equivalents, together with expected and committed cash from existing collaborations, will enable Wave to fund its operating and capital expenditure requirements to the end of 2020.

**About PRISM™**

PRISM is Wave Life Sciences' proprietary discovery and drug development platform that enables genetically defined diseases to be targeted with stereopure oligonucleotides across multiple therapeutic modalities. PRISM combines the company's unique ability to construct stereopure oligonucleotides with a deep understanding of how the interplay among oligonucleotide sequence, chemistry and backbone stereochemistry impacts key pharmacological properties. By exploring these interactions through iterative analysis of in vitro and in vivo outcomes and artificial intelligence-driven predictive modeling, the company continues to define design principles that are deployed across programs to rapidly develop and manufacture clinical candidates that meet pre-defined product profiles.

**About Wave Life Sciences**

Wave Life Sciences (NASDAQ: WVE) is a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases. Wave aspires to develop best-in-class medicines across multiple therapeutic modalities using PRISM, the company's proprietary discovery and drug development platform that enables the precise design, optimization and production of stereopure oligonucleotides. Driven by a resolute sense of urgency, the Wave team is targeting a broad range of genetically defined diseases so that patients and families may realize a brighter future. To find out more, please visit www.wavelifesciences.com and follow Wave on Twitter @WaveLifeSci.

**Forward-Looking Statements**

This press release contains forward-looking statements concerning our goals, beliefs, expectations, strategies, objectives and plans, and other statements that are not necessarily based on historical facts, including statements regarding the following, among others: the anticipated commencement, patient enrollment, data readouts and completion of our clinical trials, and the announcement of such events; the protocol, design and endpoints of our ongoing and planned clinical trials; the future performance and results of our programs in clinical trials; future preclinical activities and programs; the progress and potential benefits of our collaborations with partners; the potential of our *in vitro* and *in vivo* preclinical data to predict the behavior of our compounds in humans; our identification of future candidates and their therapeutic potential; the anticipated therapeutic benefits of our potential therapies compared to others; our ability to design compounds using multiple modalities and the anticipated benefits of that model; the anticipated benefits of our proprietary manufacturing processes and our internal manufacturing facility; our future growth and anticipated transition to a fully integrated commercial-stage company; the potential benefits of PRISM and our stereopure oligonucleotides compared with stereorandom oligonucleotides; the benefit of nucleic acid therapeutics generally; the strength of our intellectual property; and the anticipated duration of our cash runway. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including the following: our ability to finance our drug discovery and development efforts and to raise additional capital when needed; the ability of our preclinical programs to produce data sufficient to support our clinical trial applications and the timing thereof; our ability to continue to build and maintain the company infrastructure and personnel needed to achieve our goals; the clinical results of our programs, which may not support further development of product candidates; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; our effectiveness in managing future clinical trials and regulatory processes; the effectiveness of PRISM; the continued development and acceptance of oligonucleotides as a class of medicines; our ability to demonstrate the therapeutic benefits of our candidates in clinical trials, including our ability to develop candidates across multiple therapeutic modalities; our dependence on third parties, including contract research organizations, contract manufacturing organizations, collaborators and partners; our ability to manufacture or contract with third parties to manufacture drug material to support our programs and growth; our ability to obtain, maintain and protect intellectual property; our ability to enforce our patents against infringers and defend our patent portfolio against challenges from third parties; and competition from others developing therapies for similar uses, as well as the information under the caption "Risk Factors" contained in our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) and in other filings we make with the SEC from time to time. We undertake no obligation to update the information contained in this press release to reflect subsequently occurring events or circumstances.

**WAVE LIFE SCIENCES LTD.**
**UNAUDITED CONSOLIDATED BALANCE SHEETS**

*(In thousands, except share amounts)*

| | March 31, 2019 | December 31, 2018 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 287,567 | $ 174,819 |
| Current portion of accounts receivable | 10,000 | 10,000 |
| Prepaid expenses and other current assets | 17,464 | 17,454 |
| Total current assets | 315,031 | 202,273 |
| Long-term assets: | | |
| Accounts receivable, net of current portion | 50,000 | 50,000 |
| Property and equipment, net | 39,929 | 39,931 |
| Operating lease right-of-use assets | 19,333 | — |
| Restricted cash | 3,631 | 3,625 |
| Other assets | 2,688 | 111 |
| Total long-term assets | 115,581 | 93,667 |
| Total assets | $ 430,612 | $ 295,940 |
| **Liabilities, Series A preferred shares and shareholders' equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 14,577 | $ 13,089 |
| Accrued expenses and other current liabilities | 8,490 | 14,736 |
| Current portion of deferred rent | — | 115 |
| Current portion of deferred revenue | 105,891 | 100,945 |
| Current portion of lease incentive obligation | — | 1,156 |
| Current portion of operating lease liability | 2,919 | — |
| Total current liabilities | 131,877 | 130,041 |
| Long-term liabilities: | | |
| Deferred rent, net of current portion | — | 5,132 |
| Deferred revenue, net of current portion | 60,184 | 68,156 |
| Lease incentive obligation, net of current portion | — | 9,247 |
| Operating lease liability, net of current portion | 31,782 | — |
| Other liabilities | 2,039 | 2,142 |
| Total long-term liabilities | 94,005 | 84,677 |
| Total liabilities | $ 225,882 | $ 214,718 |
| Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at March 31, 2019 and December 31, 2018 | $ 7,874 | $ 7,874 |
| Shareholders' equity: | | |
| Ordinary shares, no par value; 34,255,406 and 29,472,197 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively | $ 538,414 | $ 375,148 |
| Additional paid-in capital | 42,113 | 37,768 |
| Accumulated other comprehensive income | 250 | 153 |
| Accumulated deficit | (383,921) | (339,721) |
| Total shareholders' equity | $ 196,856 | $ 73,348 |
| Total liabilities, Series A preferred shares and shareholders' equity | $ 430,612 | $ 295,940 |

**WAVE LIFE SCIENCES LTD.**
**UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**

*(In thousands, except share and per share amounts)*

| | Three Months Ended March 31, | |
|---|---|---|
| | 2019 | 2018 |
| Revenue | $ 3,026 | $ 1,422 |
| Operating expenses: | | |
| Research and development | 40,113 | 29,196 |
| General and administrative | 10,901 | 8,001 |
| Total operating expenses | 51,014 | 37,197 |
| Loss from operations | (47,988) | (35,775) |
| Other income, net: | | |
| Dividend income | 1,424 | 356 |
| Interest income, net | 11 | 7 |
| Other income, net | 2,353 | 343 |
| Total other income, net | 3,788 | 706 |
| Loss before income taxes | (44,200) | (35,069) |
| Income tax provision | — | (172) |
| Net loss | $ (44,200) | $ (35,241) |
| Net loss per share attributable to ordinary shareholders—basic and diluted | $ (1.36) | $ (1.26) |
| Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted | 32,597,158 | 27,919,063 |
| Other comprehensive income (loss): | | |
| Net loss | $ (44,200) | $ (35,241) |
| Foreign currency translation | 97 | 49 |
| Comprehensive loss | $ (44,103) | $ (35,192) |

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Exhibit 99.2

# Wave Life Sciences

## Corporate Presentation

May 10, 2019

# Forward-looking statements

This document contains forward-looking statements. All statements other than statements of historical facts contained in this document, including statements regarding possible or assumed future results of operations, preclinical and clinical studies, business strategies, research and development plans, collaborations and partnerships, regulatory activities and timing thereof, competitive position, potential growth opportunities, use of proceeds and the effects of competition are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause the actual results, performance or achievements of Wave Life Sciences Ltd. (the "Company") to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "aim," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions. The forward-looking statements in this presentation are only predictions. The Company has based these forward-looking statements largely on its current expectations and projections about future events and financial trends that it believes may affect the Company's business, financial condition and results of operations. These forward-looking statements speak only as of the date of this presentation and are subject to a number of risks, uncertainties and assumptions, including those listed under Risk Factors in the Company's Form 10-K and other filings with the SEC, some of which cannot be predicted or quantified and some of which are beyond the Company's control. The events and circumstances reflected in the Company's forward-looking statements may not be achieved or occur, and actual results could differ materially from those projected in the forward-looking statements. Moreover, the Company operates in a dynamic industry and economy. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties that the Company may face. Except as required by applicable law, the Company does not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

# Targeting genetically defined diseases with stereopure oligonucleotides

## Building fully integrated genetic medicines company led by neurology development programs

### Neuromuscular

- ***Lead clinical program:*** **Suvodirsen Phase 2/3 trial initiation expected in July 2019 for DMD (exon 51); program on development path toward US and global approvals**
- Advancing additional exon skipping candidates for DMD
- Commercialization activities underway

**100% global rights**

### CNS

- ***Lead clinical program:*** **Two Phase 1b/2a trials ongoing for Huntington's disease using differentiated allele-selective approach**
- Advancing C9orf72 candidate for ALS and FTD
- SNP3 (HD) and ATXN3 (SCA3)

**Takeda 50:50 option**

### Ophthalmology

- Initial candidate selection ongoing for inherited retinal diseases

**100% global rights**

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PRISM

**DESIGN & OPTIMIZE**


**Stereopure oligonucleotides across multiple therapeutic modalities**

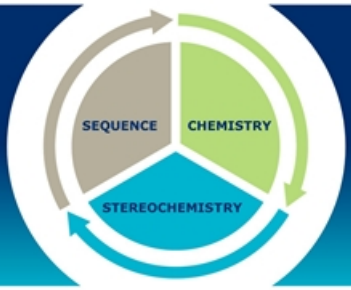
Antisense | RNAi | Splicing

PRISM

**Enables Wave to target genetically defined diseases with stereopure oligonucleotides across multiple therapeutic modalities**

**DESIGN**

Unique ability to construct stereopure oligonucleotides with one defined and consistent profile




**OPTIMIZE**

A deep understanding of how the interplay among oligonucleotide sequence, chemistry, and backbone stereochemistry impacts key pharmacological properties

**Through iterative analysis of *in vitro* and *in vivo* outcomes and artificial intelligence-driven predictive modeling, Wave continues to define design principles that are deployed across programs to rapidly develop and manufacture clinical candidates that meet pre-defined product profiles**

WAVE LIFE SCIENCES

# Designing the optimal, stereopure medicine

STANDARD OLIGONUCLEOTIDE APPROACHES

Pharmacologic properties include >500,000 permutations in every dose

↓

Impact:
Unreliable therapeutic effects
Unintended off-target effects

WAVE RATIONAL DESIGN

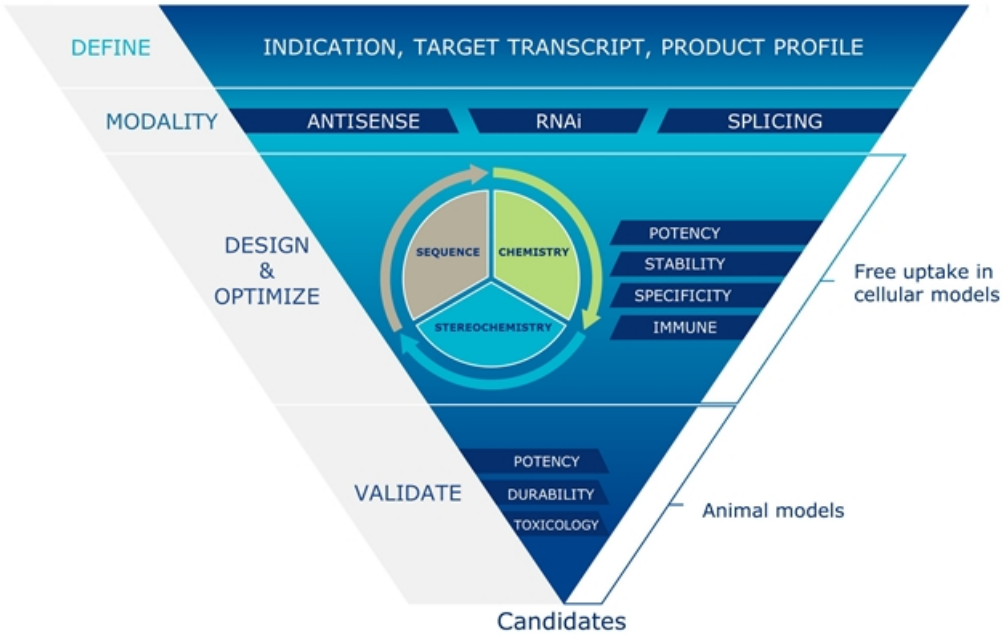
Control of stereochemistry enables the design and manufacture of oligonucleotides with one defined and consistent profile

↓

Impact:
Potential for best-in-class medicines that can address difficult-to-treat diseases

WAVE LIFE SCIENCES

# Creating a new class of oligonucleotides

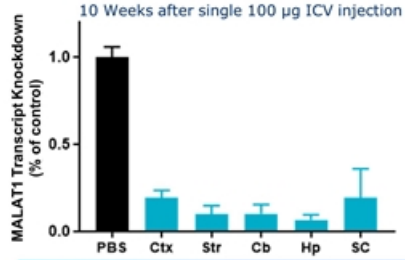



Source: Iwamoto N, et al. Control of phosphorothioate stereochemistry substantially increases the efficacy of antisense oligonucleotides. *Nat Biotechnol*. 2017;35:845-851.

# Optimizing potency and durability across multiple tissues

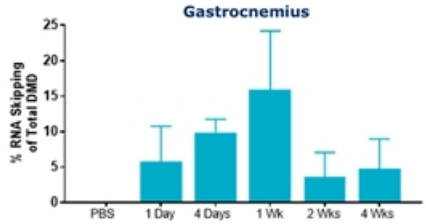
## CNS

*MALAT1* Transcript Knockdown in Mice
10 Weeks after single 100 µg ICV injection



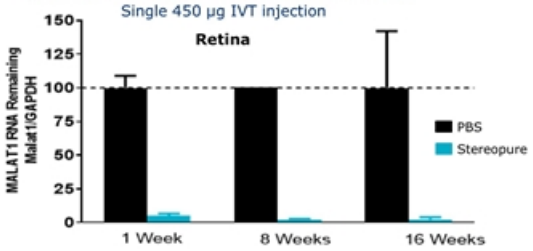

## Muscle

*DMD:* Percent Skipped Transcript in *mdx*23 Mice
Single 150 mg/kg IV injection



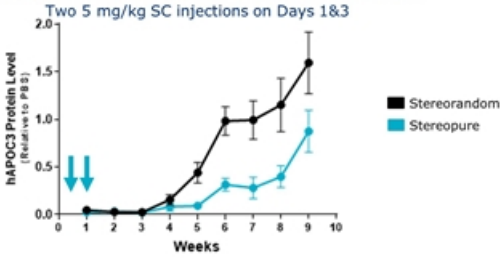

## Eye

*MALAT1* Knockdown in Non-Human Primates
Single 450 µg IVT injection




## Liver

Knockdown of Serum hAPOC3 Protein Levels in Mice
Two 5 mg/kg SC injections on Days 1&3

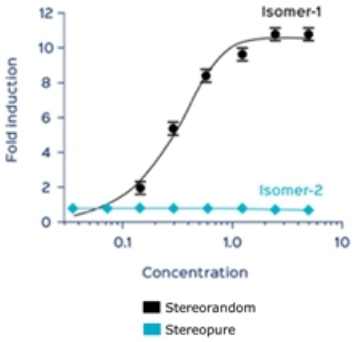



Data represented in this slide from *in vivo* studies. CNS: PBS = phosphate buffered saline; Ctx = cortex; Str = striatum; Cb = cerebellum; Hp = hippocampus; SC = spinal cord. ICV = intracerebral; IVT = intravitreal; IV = intravenous; SC= subcutaneous.

# Stereochemistry affects immune activation

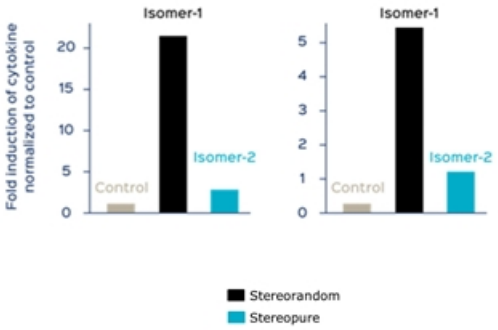
## Human TLR9 Activation

Human TLR9 activation assay with 5mC modified CpG containing MOE gapmer



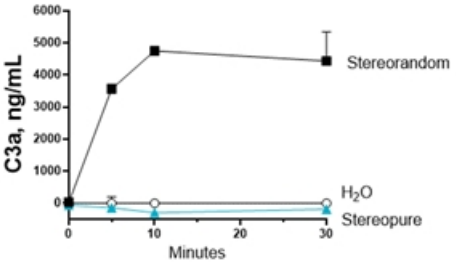

## Cytokine Induction

Cytokine induction in human PBMC assay




## Complement Activation

Complement activation in non-human primate serum assay




Data represented in this slide from *in vitro* studies. MOE = 2′-O-methoxyethylribose; PBMC = peripheral blood mononuclear cell; TLR9 = toll-like receptor 9.

# Pipeline spanning multiple modalities, novel targets

| | TARGET | ESTIMATED U.S. PREVALENCE* | MECHANISM | DISCOVERY | CANDIDATE | CLINICAL | WAVE'S COMMERCIAL RIGHTS | PARTNER |
|---|---|---|---|---|---|---|---|---|
| **MUSCLE** | | | | | | | | |
| Duchenne muscular dystrophy | Exon 51 | ~2,000 | (E) | ● | ● | OLE (Phase 1) | 100% Global | — |
| Duchenne muscular dystrophy | Exon 53 | ~1,250 | (E) | ● | ● | | 100% Global | — |
| Duchenne muscular dystrophy | Exons 44, 45, 52, 54, 55 | ~1,500 | (E) | ● | ○ | | 100% Global | — |
| Neuromuscular diseases | Multiple | | ○ | ● | ○ | | 100% Global | — |
| **CNS** | | | | | | | | |
| Huntington's disease | mHTT SNP1 | ~10k / ~35k | (A) | ● | ● | Phase 1b/2a | 50% Global | Takeda |
| Huntington's disease | mHTT SNP2 | ~10k / ~35k | (A) | ● | ● | Phase 1b/2a | 50% Global | Takeda |
| Huntington's disease | mHTT SNP3 | ~ 8k / ~ 30k | (A) | ● | ○ | | 50% Global | Takeda |
| Amyotrophic lateral sclerosis | C9orf72 | ~1,800 | (A) | ● | ● | | 50% Global | Takeda |
| Frontotemporal dementia | C9orf72 | ~7,000 | (A) | ● | ● | | 50% Global | Takeda |
| Spinocerebellar ataxia 3 | ATXN3 | ~4,500 | (S) | ● | ● | | 50% Global | Takeda |
| CNS diseases | Multiple† | | ○ | ● | ○ | | Milestones & Royalties | Takeda |
| **OPHTHALMOLOGY** | | | | | | | | |
| Retinal diseases | Multiple | ~10,000 | ○ | ● | ○ | | 100% Global | — |
| **HEPATIC** | | | | | | | | |
| Metabolic liver diseases | Multiple | | (S) | ● | ○ | | Milestones & Royalties | Pfizer |

(S) = silencing. (A) = allele-specific silencing. (E) = exon skipping. OLE = Open-label extension.

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*Estimates of U.S. prevalence and addressable population by target based on publicly available data and are approximate; for Huntington's disease, numbers approximate manifest and pre-manifest populations, respectively.
† During a four-year term, Wave and Takeda may collaborate on up to six preclinical targets at any one time.

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# Duchenne Muscular Dystrophy (DMD)

# DMD: a progressive, fatal childhood disorder

- Fatal, X-linked genetic neuromuscular disorder characterized by progressive, irreversible loss of muscle function, including heart and lung
- Genetic mutation in dystrophin gene prevents the production of dystrophin protein, a critical component of healthy muscle function
- Symptom onset in early childhood; one of the most serious genetic diseases in children worldwide
- Current disease modifying treatments have demonstrated minimal dystrophin expression and clinical benefit has not been established
- Impacts 1 in every 5,000 newborn boys each year; 20,000 new cases annually worldwide

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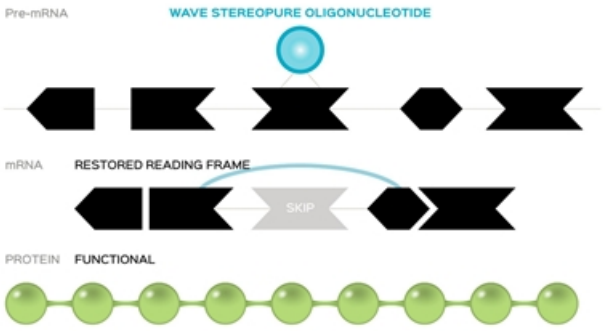
Source: Parent Project Muscular Dystrophy. About Duchenne & Becker muscular dystrophy. Available at: https://www.parentprojectmd.org/care/for-healthcare-providers/. Accessed: November 2, 2018.

# Wave approach: stereopure exon skipping oligonucleotide

## Exon skipping




## Potential benefits of an oligonucleotide approach to treating a lifelong disease

- Chronic administration may better address high muscle cell turnover and need for broad and durable distribution
- Entry into cells, including progenitor cells, via free-uptake
- Production of functional dystrophin protein, not micro-dystrophin
- Scalable manufacturing

**Exon skipping with stereopure oligonucleotides has the potential to enable production of meaningful levels of functional dystrophin which is expected to result in therapeutic benefit**

Sources: Arnett ALH, et al. *Mol Ther Methods Clin Dev.* 2014;1:14038. doi:10.1038/mtm.2014.38. Counsell JR, et al. *Sci Rep.* 2017;7:79. doi: 10.1038/s41598-017-00152-5. Duan D. *Mol Ther.* 2018;25:2337-2356. Martinsen B, Dreyer P. *Open Nurs Jrnl.* 2016;10:131-138. Stitelman DH, et al. *Mol Ther Methods Clin Dev.* 2014;1:14040. doi:10.1038/mtm.2014.40.

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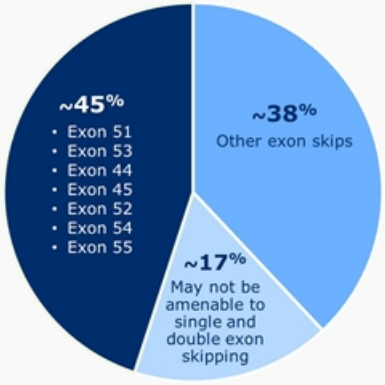
# Building a portfolio to transform the care of DMD

**Suvodirsen targeting exon 51**
- Phase 2/3 trial expected to commence in July 2019 for global regulatory submissions
- Potential FDA accelerated approval filing in 2H 2020, pending positive clinical dystrophin expression data

**WVE-N531 targeting exon 53**
- Topline clinical data expected in 2H 2020

**Advancing candidate development for exons 44, 45, 52, 54, 55**
- Early leads demonstrated similar *in vitro* exon skipping efficiency as suvodirsen and WVE-N531

Percentage of patients with DMD amenable to exon skipping therapeutic approach




**Initiating commercialization activities in anticipation of first potential launch in US**

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Sources: Aartsma-Rus A, et al. *Hum Mutat.* 2009;30:293-299. Bladen CL, et al. *Hum Mutat.* 2015;36:395–402.

# Suvodirsen: Path towards US and global approvals


## Phase 1

- Phase 1 single ascending dose clinical trial
- Based on *in vitro* and *in vivo* preclinical studies and Phase 1 clinical results, two suvodirsen doses selected for Phase 2/3 clinical trial
- **Study complete**

## Open-label extension (OLE)

- Multi-dose, open-label study with patients from Phase 1 clinical trial
- Data will be an important component of submission for accelerated approval in US
- **On track to deliver interim analysis of dystrophin expression in 2H 2019**

## Phase 2/3 DYSTANCE 51

- Phase 2/3 clinical trial to assess clinical efficacy and dystrophin expression
- Efficacy and safety data to serve as basis of regulatory submissions globally
- Selected for FDA pilot program for complex innovative trial designs
- **Expect to initiate in July 2019**

**2H 2020:** Potential FDA accelerated approval filing in exon 51 amenable DMD

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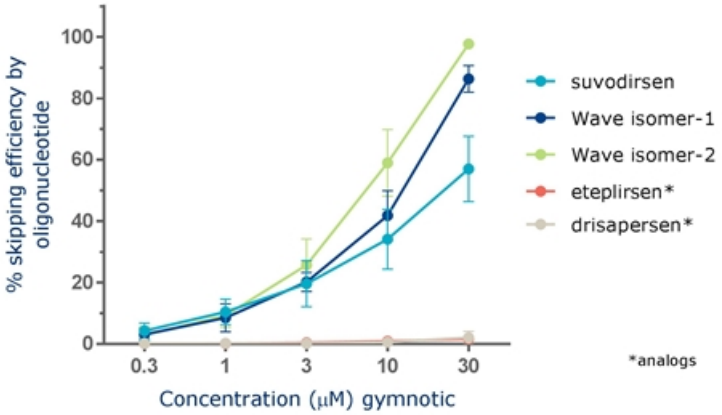
Suvodirsen formerly named WVE-210201

# Exon 51: improved skipping efficiency

- RNA skipping determined by quantitative RT-PCR
- Wave isomers demonstrated a dose-dependent increase in skipping efficiency *in vitro*
- Free uptake at 10uM concentration of each compound with no transfection agent
- Same foundational stereopure chemistry for Wave isomers; individually optimized to select candidate

Dose Response on Skipping Efficiency
(mRNA, *in vitro*) (4 days)

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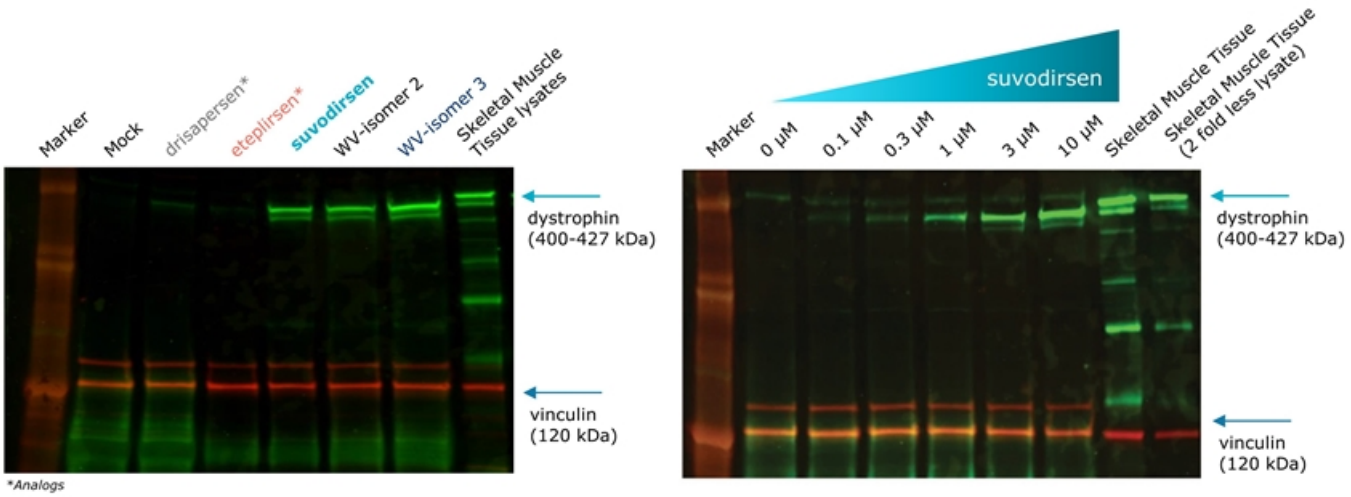
Experimental conditions: Free uptake of ASO in human DMD myoblast cells. Skipping quantified by TaqMan assay.

# Exon 51: increased dystrophin restoration




Dystrophin protein restoration *in vitro* was quantified to be between **50-100% of normal** skeletal muscle tissue lysates, as compared to about 1% by drisapersen and eteplirsen analogs

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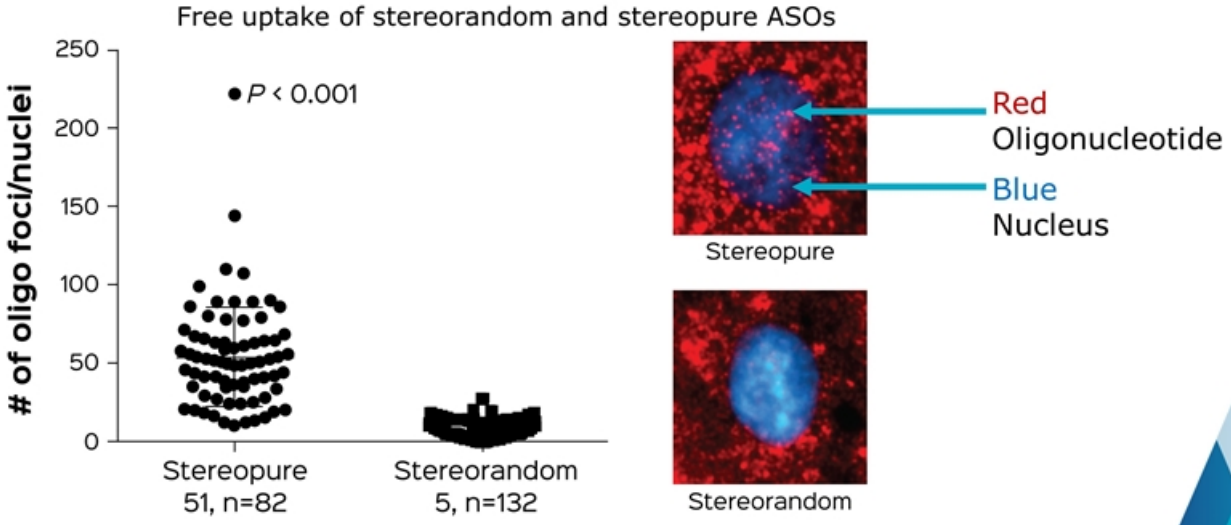
Experimental conditions: DMD protein restoration by Western Blot in patient-derived myotubes with clear dose effect. Free uptake at 10 µM concentration of each compound with no transfection agent.

# Exon 51: improved oligonucleotide uptake in the nucleus where splicing occurs

Stereopure oligonucleotides are designed to readily enter the nuclei of cells under free-uptake conditions, which approximates natural delivery in the body




Experimental conditions: Free uptake of ASOs in 18 hour differentiating human DMD myoblasts (Δ48-50).

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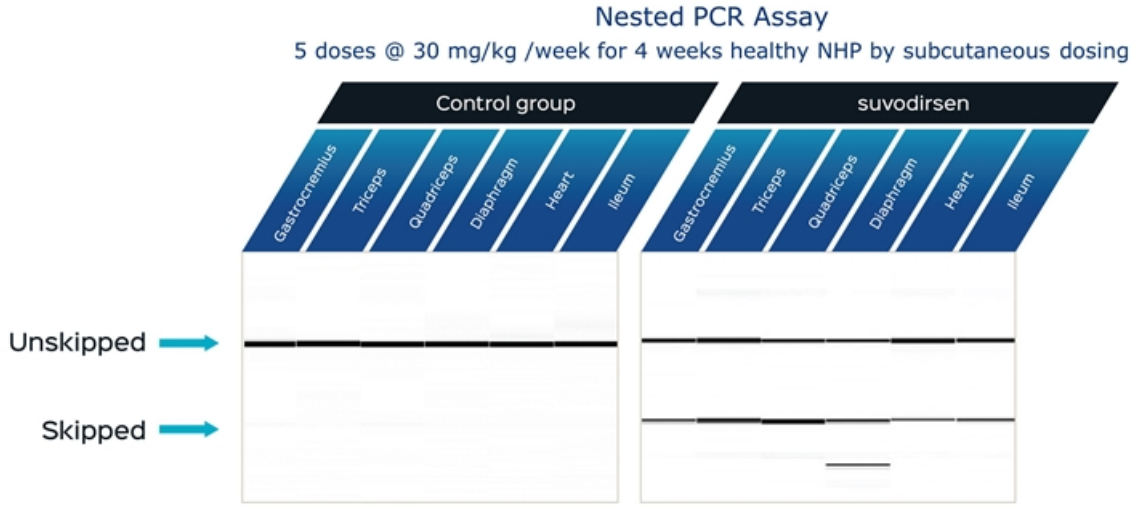
# Exon 51: *in vivo* target engagement of suvodirsen in healthy non-human primate

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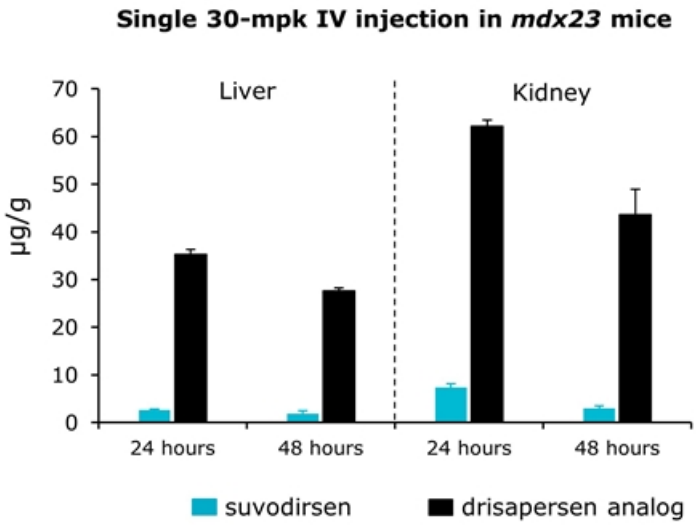
Experimental conditions: Muscle tissues were collected 2 days after the last dose and fresh frozen. Total RNAs were extracted with phenol/chloroform and converted to cDNA using high capacity kit. Nested PCR assay was performed and analyzed by fragment analyzer.

# Exon 51: no apparent tissue accumulation observed

- Standard oligonucleotides tend to accumulate in liver and kidney
- Wave rationally designed oligonucleotides optimized to allow compound to clear more effectively
- Suvodirsen demonstrated wide tissue distribution in dose dependent fashion
- No apparent accumulation observed after multiple doses




Experimental conditions: *Mdx23* mice received a single 30-mg/kg intravenous bolus injection of suvodirsen or drisapersen analog (n=3/group), and sacrificed 24 or 48 hours post dose. Oligo quantifications in tissues were performed using hybridization ELISA assay.

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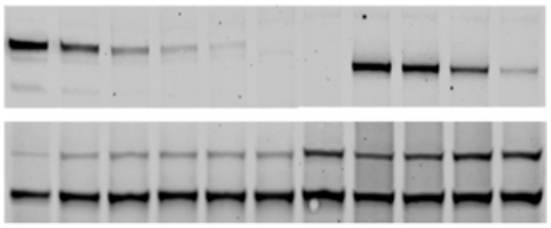
# Exon 53: WVE-N531 *in vitro* dose-dependent dystrophin restoration

Dystrophin protein restoration of up to 71%

**Western Blot normalized to primary healthy human myoblast lysate**

| Standard Curve (% WT lysate in D45-52 lysate) | | | | | | D45-52 Cells | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|
| | | | | | | Mock | WVE-N531 | | | | |
| | | | | | | 0 | 10 | 3.3 | 1.1 | 0.3 | Conc [uM] |
| 100% | 50% | 25% | 12% | 6% | 0% | 0% | 71% | 65% | 37% | 9.5% | % Dystrophin |




- Free uptake for 6 days in differentiation media with no transfection agent and no peptide conjugated to the oligonucleotide
- Wave stereopure exon 53 candidate demonstrated a dose-dependent increase in dystrophin restoration in DMD patient-derived myoblasts

Topline clinical data expected in 2H 2020

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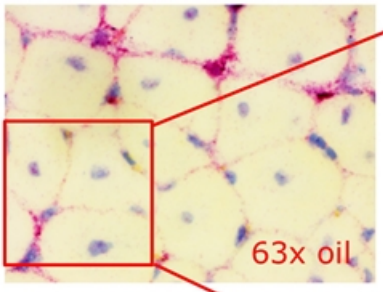
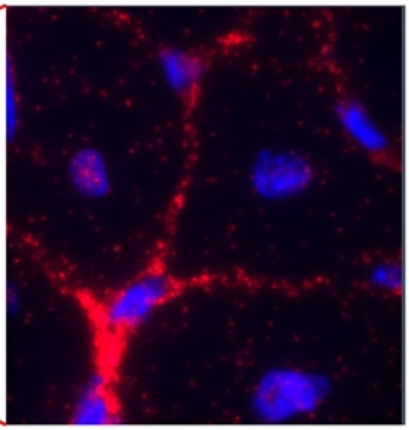
Experimental conditions: D45-52 patient myoblasts were treated with oligonucleotide for 6d under free-uptake conditions in differentiation media. Protein harvested in RIPA buffer and dystrophin restoration analyzed by Western Blot. Signal normalized to vinculin loading control and to primary healthy human myotube lysate (pooled from four donors) forming a standard curve in d45-52 cell lysate.

# Exon 53: targeting oligonucleotide rapidly distributes to muscle within 24 hours after injection



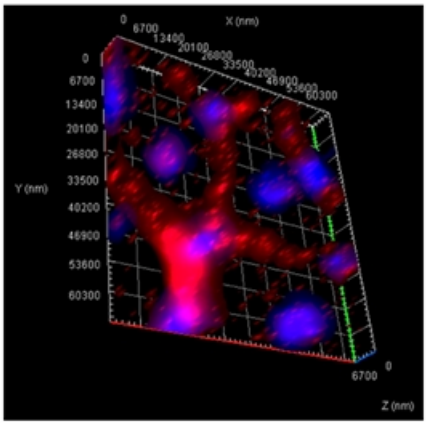

Bright field view

**Nucleus**: Hematoxylin; Light Blue
**Wave oligo:** ViewRNA, Fast Red



Fluorescence channel view

**Nucleus**: Hoechst33342; Blue
**Wave oligo:** Fast Red/Cy3; Pink Red




Z Stack view

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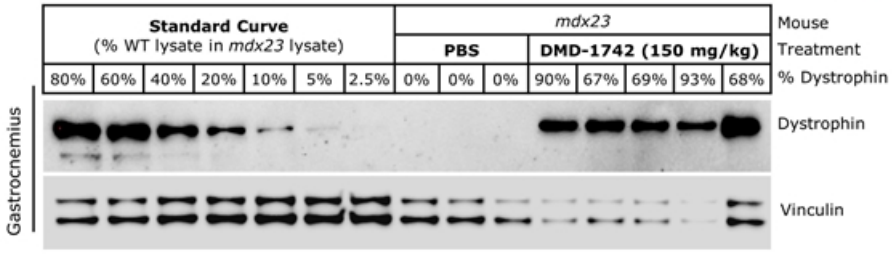
Data derived from *in vivo* preclinical research.

Experimental conditions: A single dose of stereopure oligonucleotide 30 mg/kg IV was administered to *mdx* 23 mice. Tissues collected 24 hours post dose and ASO was detected in muscles using ViewRNA.

# *In vivo mdx23* dystrophin protein with oligonucleotides

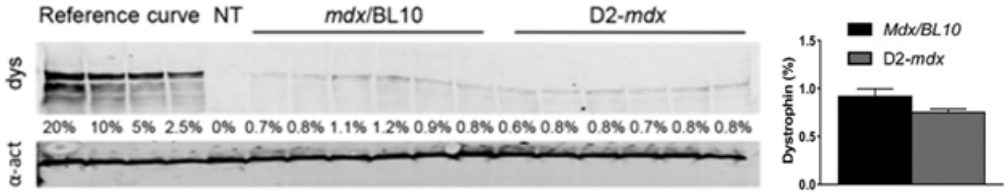
***In vivo* dystrophin protein restoration (stereopure surrogate, 150 mg/kg, 4 weekly IV doses)**




- 70 – 90% dystrophin restoration
- 87% reduction in creatine kinase (CK) levels

Published literature

***In vivo* dystrophin protein restoration (drisapersen surrogate, 200 mg/kg, 8 weekly IV doses)**




- Less than 1.5% dystrophin restoration in two separate studies[1,2]
- No reduction in CK levels[1]

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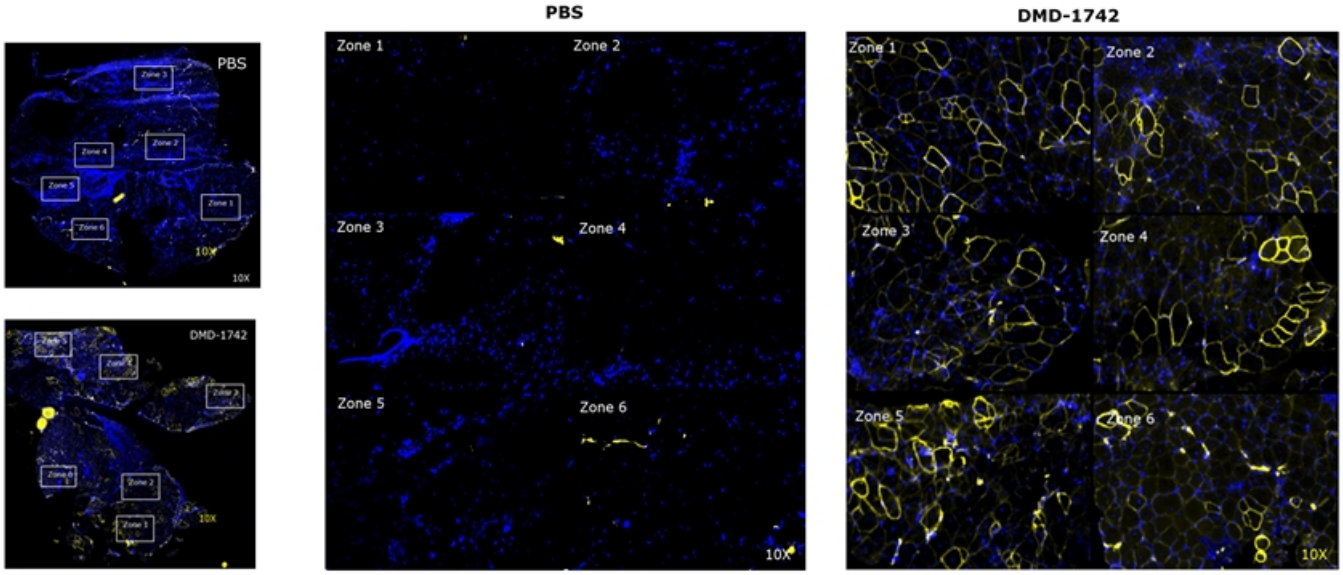
NT = nontreated *mdx* mouse; *mdx*/BL10 = mdx mouse in C57BL/10ScSnJ background; D2-*mdx* = mdx mouse crossed to DBA/2A background resulting in more severely affected model; CK = creatine kinase
Experimental conditions (stereopure surrogate): Tissues collected 96 hours post final dose. Protein expression determined by western Blot.
1. Experimental conditions (drisapersen surrogate): Tissues collected 1 week after the last injection. Protein expression determined by western blot.
van Putten M, Tanganyika-de Winter C, Bosgra S, Aartsma-Rus A. Nonclinical Exon Skipping Studies with 2'-O-Methyl Phosphorothioate Antisense Oligonucleotides in mdx and mdx-utrn-/- Mice Inspired by Clinical Trial Results. *Nucleic Acid Ther.* 2019 Apr;29(2):92-103.
2. Molecular Therapy – Nucleic Acids (2014) 3, e148

# Single dose of surrogate results in restoration of dystrophin in muscle fibers

## Immunohistochemistry of dystrophin in gastrocnemius in *mdx23* mice at 4 weeks

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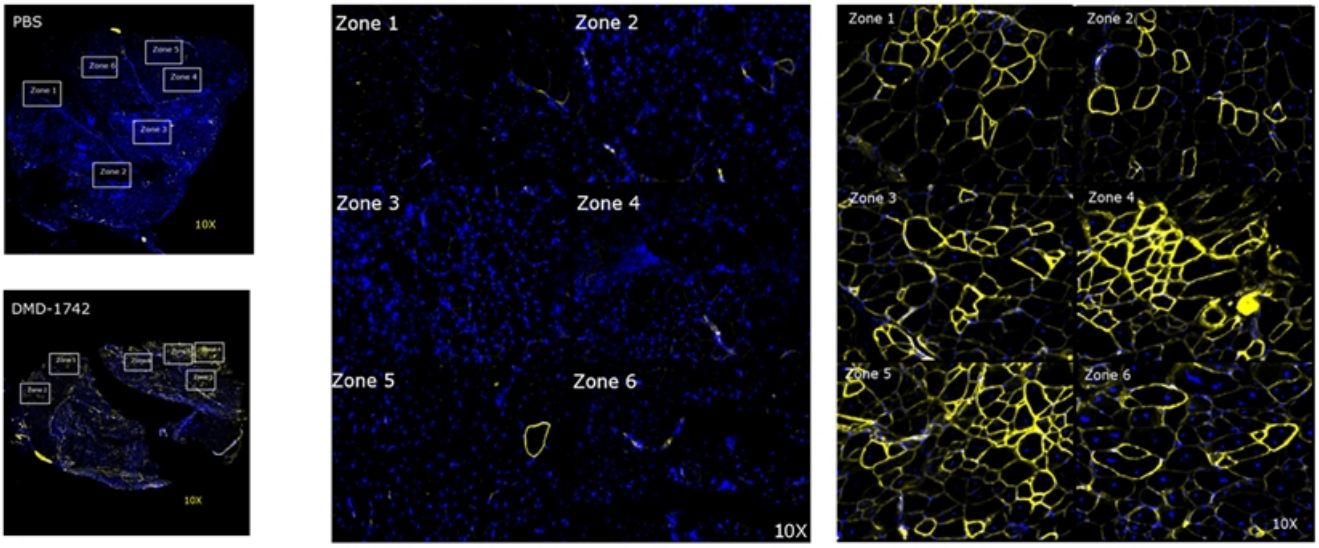
Experimental conditions: *mdx23* mice received a single IV injection of PBS or DMD-1742 (150 mg/kg).
Immunohistochemistry: Blue: Nuclei, Hoechest; Yellow: Rabbit anti-Dystrophin(#ab15277) 1:400 diluent, 555/Cy3, Cy3 staining is represented by the yellow color.
10X magnification.

# Multiple doses of surrogate result in further restoration of dystrophin in muscle fibers

## Immunohistochemistry of dystrophin in gastrocnemius in *mdx23* mice at 4 weeks




Experimental conditions: *mdx23* mice received 4 weekly IV injections of PBS or DMD-1742 (150 mg/kg).
Immunohistochemistry: Blue: Nuclei, Hoechest; Yellow: Rabbit anti-Dystrophin(#ab15277) 1:400 diluent, 555/Cy3, Cy3 staining is represented by the yellow color.
10X magnification.

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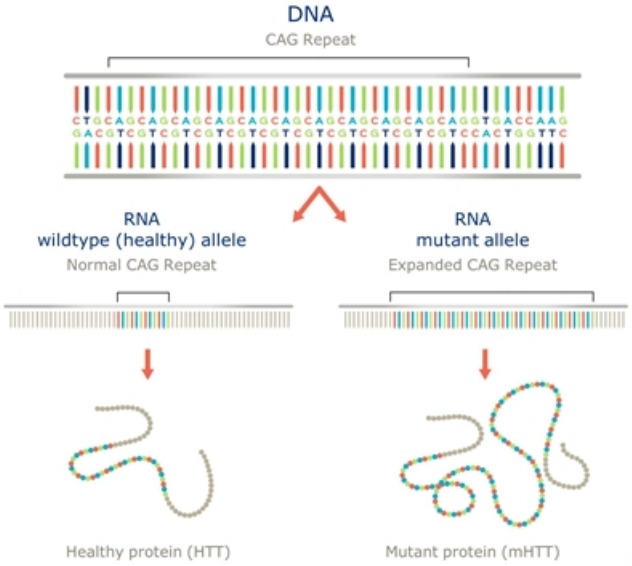
# Huntington's Disease

# Huntington's disease: a hereditary, fatal disorder

- Autosomal dominant disease, characterized by cognitive decline, psychiatric illness and chorea; fatal
- No approved disease-modifying therapies
- Expanded CAG triplet repeat in HTT gene results in production of mutant huntingtin protein (mHTT); accumulation of mHTT causes progressive loss of neurons in the brain
- Wildtype (healthy) HTT protein critical for neuronal function; suppression may have detrimental long-term consequences
- 30,000 people with Huntington's disease in the US; another 200,000 at risk of developing the condition

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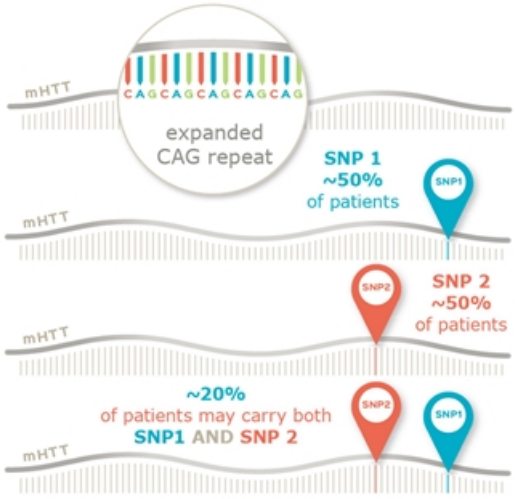
Sources: Auerbach W, et al. *Hum Mol Genet*. 2001;10:2515-2523. Dragatsis I, et al. *Nat Genet*. 2000;26:300-306. Leavitt BR, et al. *J Neurochem*. 2006;96:1121-1129. Nasir J, et al. *Cell*. 1995;81:811-823. Reiner A, et al. *J Neurosci*. 2001;21:7608-7619. White JK, et al. *Nat Genet*. 1997;17:404-410. Zeitlin S, et al. *Nat Genet*. 1995;11:155-163. Carroll JB, et al. *Mol Ther*. 2011;19:2178-2185.
Huntington Disease Society of America (HDSA). What is Huntington's disease? Available at: http://hdsa.org/what-is-hd/. Accessed: November 2, 2018.

# Wave approach: novel, allele-specific silencing

- Utilize association between single nucleotide polymorphisms (SNPs) and genetic mutations to specifically target errors in genetic disorders, including HD
- Allele-specificity possible by targeting SNPs associated with expanded long CAG repeat in mHTT gene
- Approach aims to lower mHTT transcript while leaving healthy HTT relatively intact
- Potential to provide treatment for up to 70% of HD population (either oligo alone could address approximately 50% of HD population)




Total: Due to overlap, an estimated **~70%** of the total HD patient population carry SNP 1 and/or SNP 2

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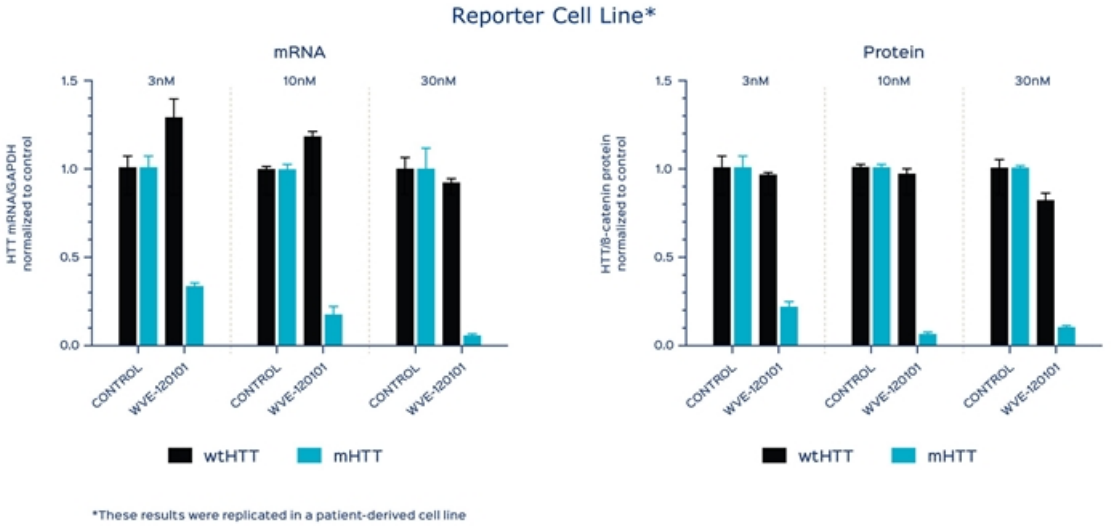
Source: Kay, et al. Personalized gene silencing therapeutics for Huntington disease. *Clin Genet.* 2014;86:29–36.

# Two simultaneous Phase 1b/2a clinical trials

- PRECISION-HD is a global clinical program consisting of the PRECISION-HD1 trial evaluating WVE-120101 targeting SNP1 and the PRECISION-HD2 trial evaluating WVE-120102 targeting SNP2
  - Two parallel, multicenter, double-blind, randomized, placebo-controlled Phase 1b/2a clinical trials for WVE-120101 and WVE-120102, administered intrathecally, with single-ascending dose and multiple-ascending dose portions
  - Primary objective: Assess safety and tolerability of intrathecal doses in early manifest HD patients
  - Key additional objectives: Measurement of total HTT and mHTT; exploratory pharmacokinetic (PK), pharmacodynamic (PD), clinical and MRI endpoints
  - Key inclusion criteria: age ≥25 to ≤65, stage I or II HD who have screened positively for the presence of SNP1 or SNP2
  - Expected to enroll approximately 50 patients per trial
- Open-label extension (OLE) study planned to allow for continued dosing and clinical assessments
  - To include patients previously in the Phase 1b/2a clinical trials
  - Assessments of safety, tolerability, PK, PD, MRI and efficacy using validated clinical outcome measures
- Intend to explore efficacy in early manifest and pre-manifest HD patient populations

Phase 1b/2a readout expected by YE 2019

# Selective reduction of mHTT mRNA & protein

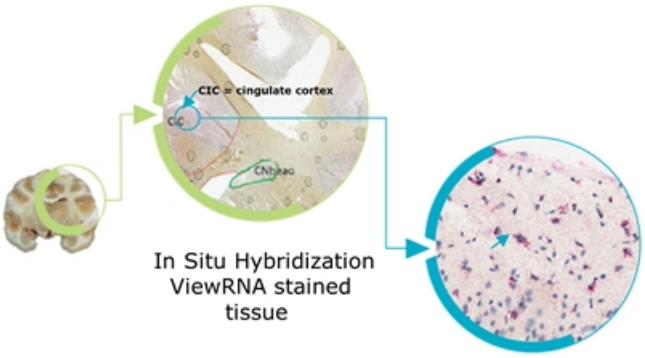



*These results were replicated in a patient-derived cell line

Source: Meena, Zboray L, Svrzikapa N, et al. Selectivity and biodistribution of WVE-120101, a potential antisense oligonucleotide therapy for the treatment of Huntington's disease. Paper presented at: 69th Annual Meeting of the American Academy of Neurology; April 28, 2017; Boston, MA.

# Demonstrated delivery to brain tissue

- WVE-120101 and WVE-120102 distribution in cynomolgus non-human primate brain following intrathecal bolus injection

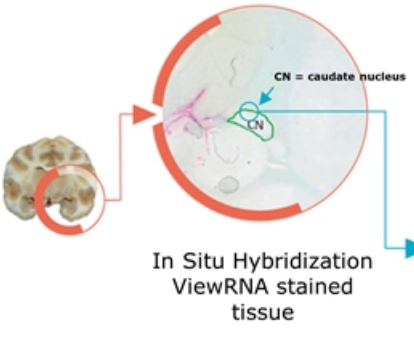



In Situ Hybridization ViewRNA stained tissue

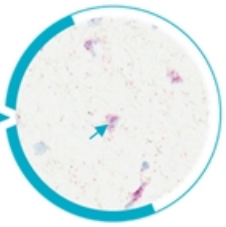


Red dots are WVE-120101 oligonucleotide

Arrow points to nuclear and perinuclear distribution of WVE- 120101 in cingulate cortex




In Situ Hybridization ViewRNA stained tissue



Red dots are WVE-120102 oligonucleotide

Arrow points to nuclear and perinuclear distribution of WVE-120102 in caudate nucleus

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Source: Meena, Zboray L, Svrzikapa N, et al. Selectivity and biodistribution of WVE-120101, a potential antisense oligonucleotide therapy for the treatment of Huntington's disease. Paper presented at: 69th Annual Meeting of the American Academy of Neurology; April 28, 2017; Boston, MA.

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# C9orf72

## Amyotrophic Lateral Sclerosis (ALS)

## Frontotemporal Dementia (FTD)

# C9orf72: a critical genetic risk factor

- C9orf72 gene provides instructions for making protein found in various tissues, with abundance in nerve cells in the cerebral cortex and motor neurons
- C9orf72 genetic mutations are the strongest genetic risk factor found to date for the more common, non-inherited (sporadic) forms of Amyotrophic Lateral Sclerosis (ALS) and Frontotemporal Dementia (FTD); GGGGCC repeat drives the formation and accumulation of dipeptide repeat proteins that accumulate in brain tissue
- First pathogenic mechanism identified to be a genetic link between familial (inherited) ALS and FTD
- Most common mutation identified associated with familial ALS and FTD
- Availability of dipeptide biomarker in CSF has potential to accelerate drug development


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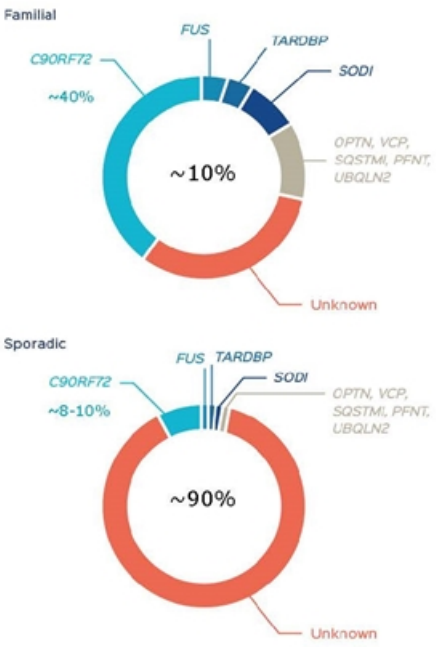
Source: DeJesus-Hernandez M, Mackenzie IR, Boeve BF, et al. *Neuron.* 2011;72:245-256. Renton AE, Majounie E, Waite A, et al. *Neuron.* 2011;72:257-268.

# Amyotrophic lateral sclerosis

- Fatal neurodegenerative disease characterized by the progressive degeneration of motor neurons in the brain and spinal cord
- Affects approximately 15,000-20,000 people in the US with a median survival of three years
- C9orf72 is present in approximately 40% of familial ALS and 8-10% of sporadic ALS; currently the most common demonstrated mutation related to ALS, far more so than SOD1 or TDP-43
- Pathogenic transcripts of the C9orf72 gene contain hundreds to thousands of hexanucleotide repeats compared to 2-23 in wild-type transcripts; dominant trait with high penetrance

**Clinical development expected to initiate in 2H 2020**

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Source: Renton AE, Chiò A, Traynor BJ. State of play in amyotrophic lateral sclerosis genetics. *Nat Neurosci.* 2014;17:17–23.

# Frontotemporal dementia

- Progressive neuronal atrophy with loss in the frontal and temporal cortices characterized by personality and behavioral changes, as well as gradual impairment of language skills
- Affects approximately 55,000 people in the US
- Second most common form of early-onset dementia after Alzheimer's disease in people under the age of 65
- Up to 50% of FTD patients have a family history of dementia, many inheriting FTD as an autosomal dominant trait with high penetrance
- Pathogenic transcripts of the C9orf72 gene contain hundreds to thousands of hexanucleotide repeats compared to 2-23 in wild-type transcripts


Clinical development expected to initiate in 2H 2020

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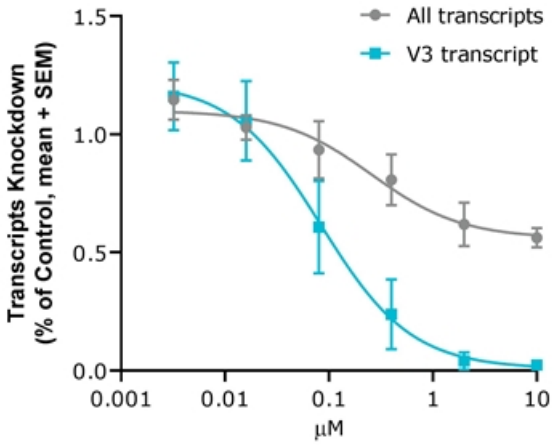
Sources: Stevens M, et al. Familial aggregation in frontotemporal dementia. *Neurology.* 1998;50:1541-1545. Majounie E, et al. Frequency of the C9orf72 hexanucleotide repeat expansion in patients with amyotrophic lateral sclerosis and frontotemporal dementia: a cross-sectional study. *Lancet Neurol.* 2012;11:323-330.

# WVE-C092 demonstrated selective and potent silencing of expanded C9orf72 repeat transcripts

## WVE-C092 preferentially reduces repeat-containing V3 transcripts




## Stereochemistry and chemistry optimization improves potency

| | $IC_{50}$ (nM) |
|---|---|
| **WVE-C092** | **84** |
| WVE-3972-01 | 411 |
| Stereorandom ASO | 845 |

**10-fold**

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*in vitro* experimental methods: C9 ALS patient derived MNs were treated with ASO gymnotically (free-uptake) for 1 week. Cell were harvested with Trizol reagent for RNA extraction. Taqman qPCR assays were used to detect V3 and all V.

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# Ophthalmology

# Building a portfolio for inherited retinal diseases

## Inherited retinal diseases (IRDs)

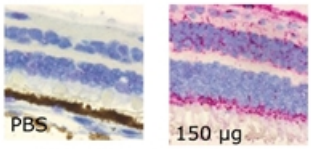
- Rare eye disorders caused by genetic mutations leading to progressive vision loss
- No approved therapies for almost any IRDs
- Approximately 200,000 affected in the U.S. and millions world-wide

## Wave opportunity

- Oligonucleotides allow for intravitreal (IVT) injection; targeting twice per year dosing
- Stereopure oligonucleotides open up novel strategies in both dominant and recessive IRDs; potential for potent and durable effect with low immune response
- Established imaging markers, easily identifiable patient population and historical ophthalmology trial success rates suggest clear path to market

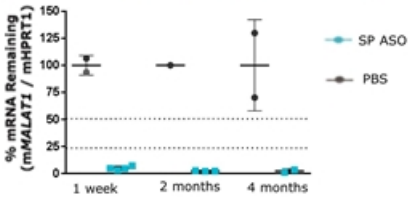
Single IVT injection of stereopure oligonucleotide to NHP results in distribution throughout all layers of the retina and potent, extended duration of effect

**Broad Distribution**
One Week Post-Dose




MALAT1 oligonucleotide detected using ViewRNA assay; pink = oligonucleotide

**>95% Knockdown**
in Retina Tissue




| Genetic target | Inherited retinal disease | US Population Addressable by Wave Approach |
|---|---|---|
| RHO P23H | Retinitis pigmentosa | ~1,800 |
| USH2A | Usher syndrome 2A | ~5,000 |
| ABCA4 | Stargardt disease | ~2,000 |
| CEP290 | Leber congenital amaurosis 10 | ~1,000 |

**Initial candidate expected in 2H 2019**

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Sources: Daiger S, et al. *Clin Genet*. 2013;84:132-141. Wong CH, et al. *Biostatistics*. 2018; DOI: 10.1093/biostatistics/kxx069. Athanasiou D, et al. *Prog Retin Eye Res*. 2018;62:1–23. Daiger S, et al. *Cold Spring Harb Perspect Med*. 2015;5:a017129. Verbakel S, et al. *Prog Retin Eye Res*. 2018:66:157-186.

# Anticipated Upcoming Wave Milestones

## Neuromuscular

- **July 2019:** Initiation of DYSTANCE 51 Phase 2/3 clinical trial for suvodirsen in DMD (exon 51)
- **2H 2019:** Interim dystrophin data readout for suvodirsen from OLE in DMD (exon 51)
- **2H 2020:** Accelerated approval filing for suvodirsen in DMD (exon 51) in US, pending positive clinical dystrophin expression data
- **2H 2020:** Topline clinical data for WVE-N531 in DMD (exon 53)

## CNS

- **By YE 2019:** Topline data readout from PRECISION-HD Phase 1b/2a trials in Huntington's disease
- **2H 2020:** Initiation of clinical development of WVE-C092 (C9orf72) in ALS and FTD

## Ophthalmology

- **2H 2019:** Selection of initial development candidate for inherited retinal disease

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# Realizing the potential of genetic medicines


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