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): March 1, 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:

☐ 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. ☐

ITEM 2.02 Results of Operations and Financial Condition.

On March 1, 2019, Wave Life Sciences Ltd. (the "Company") announced its financial results for the quarter and year ended December 31, 2018. 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 March 1, 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 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 March 1, 2019
99.2	Corporate Presentation of Wave Life Sciences Ltd. dated March 1, 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: March 1, 2019

Wave Life Sciences Reports Fourth Quarter and Full-Year 2018 Financial Results and Provides Business Update

Outlines plans for building a fully integrated genetic medicines company

CAMBRIDGE, Mass., March 1, 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 reported financial results for the fourth quarter and full year ended December 31, 2018 and outlined its plans for building a fully integrated company.

“Our achievements throughout 2018 established a strong foundation upon which we are building a world-class and fully integrated genetic medicines company,” said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. “For 2019, we will remain keenly focused on broadening our pipeline and preparing to launch suvodirsen for the treatment of Duchenne muscular dystrophy in boys who are amenable to exon 51 skipping. Realizing our long-term commitment to those affected by rare genetic diseases will require us to continue to advance our ongoing clinical programs, meaningfully expand into new therapeutic areas such as ophthalmology and continue to invest in and evolve our robust and innovative discovery and drug development platform.”

Building a Fully Integrated Genetic Medicines Company

Wave is committed to building upon its discovery, clinical development and manufacturing capabilities and continuing its rapid transformation to become a fully integrated genetic medicines company aspiring to deliver best-in-class medicines. To achieve this ambition, the company is focused on the following priority objectives:

- **Urgently advancing suvodirsen (WVE-210201) toward global commercial launches, including a potential accelerated approval in the United States:** Currently, suvodirsen, the company’s investigational therapy for boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping, is being studied in an ongoing open-label extension (OLE) study and Wave expects to deliver an interim analysis of dystrophin expression from this study in the second half of 2019. Data from the OLE interim analysis are intended to be an important component of the company’s submission to the U.S. Food and Drug Administration (FDA) for accelerated approval in the United States. Also this year, Wave anticipates initiating a global, placebo-controlled Phase 2/3 efficacy and safety clinical trial of suvodirsen. The planned Phase 2/3 trial, which is the first program to be selected for the FDA pilot program for complex innovative trial designs (CID), is designed to measure clinical efficacy and dystrophin expression, and Wave intends to use the results of this trial to seek regulatory approvals globally.

  Wave also intends to make initial investments in commercial capabilities to support the company’s transition to a fully integrated, commercial-stage genetic medicines company.

- **Delivering on the PRECISION-HD clinical trials and progressing the pipeline in neuromuscular and central nervous system (CNS) diseases:** The 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, remains on track to deliver topline data in the first half of 2019. 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.

  In addition, Wave is developing programs in neuromuscular diseases, including WVE-N531 targeting DMD exon 53 and programs targeting DMD exons 44, 45, 52, 54 and 55, as well as conducting research to identify potential targets for other neuromuscular diseases.

  The company is also advancing WVE-3972-01 in amyotrophic lateral sclerosis and frontotemporal dementia and a lead candidate for spinocerebellar ataxia 3.

  As part of its collaboration with Takeda, Wave is advancing preclinical programs for the treatment of additional CNS diseases, including Alzheimer's disease and Parkinson's disease. Under the terms of the agreement, Wave may collaborate with Takeda on up to six preclinical programs at any one time, during a four-year term. Takeda is funding at least $60 million of Wave's preclinical activities and will reimburse Wave for agreed-upon additional expenses. Takeda is entitled to exclusively license multiple preclinical programs during the term. Wave is eligible for precommercial and commercial milestone payments as well as tiered high single-digit to mid-teen royalty payments on global commercial sales of each licensed program.

- **Selecting first candidate in ophthalmology:** Wave is advancing stereopure oligonucleotides for the potential treatment of inherited retinal diseases. Wave's research in ophthalmology is assessing four inherited retinal diseases, which typically begin in childhood or adolescence and commonly lead to progressive vision loss: retinitis pigmentosa due to a P23H mutation in the RHO gene, Stargardt disease, Usher syndrome type 2A and Leber congenital amaurosis 10. Wave data presented in October 2018 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.

- **Evolving Wave's discovery and drug development platform, PRISM™:** The company recently branded its proprietary discovery and drug development platform as PRISM. 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 leveraging artificial intelligence-driven predictive modelling, the company is continuing to explore these interactions to develop an expanding set of design principles that can be applied to a variety of programs across various therapeutic areas.

Fourth Quarter Highlights and Business Update

- **Suvodirsen Phase 2/3 trial chosen for FDA complex innovative trial designs pilot program**

  In January 2019, Wave announced that the planned Phase 2/3 efficacy and safety trial of suvodirsen was selected for the FDA CID 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. As a participant in the program, the company will also have additional opportunities to meet with FDA staff to discuss the design elements of the trial, including the use of Bayesian methods to adapt the trial and allow for more efficient and productive clinical determinations.

- **Suvodirsen Phase I results support initiation of Phase 2/3 clinical trial for suvodirsen in DMD**

  In December 2018, Wave announced that the safety and tolerability data from the suvodirsen Phase 1 clinical trial in boys with DMD who are amenable to exon 51 skipping support the initiation of a Phase 2/3 clinical trial. Based on results from the Phase 1 clinical trial and pending final analysis, Wave selected a dose for its planned Phase 2/3 clinical trial of suvodirsen. The company plans to present the results from the Phase 1 clinical trial at upcoming scientific meetings.

- **Hepatic collaboration with Pfizer moving toward candidate selection**

  In 2018, Pfizer completed the selection of targets under the terms of the collaboration agreement between the two companies to develop genetically targeted therapies for the treatment of metabolic hepatic diseases, such as nonalcoholic steatohepatitis. Pfizer has selected five targets, the maximum number of targets permitted under terms of the agreement. Wave is currently advancing programs toward the selection of clinical candidates, at which point Pfizer may elect to exclusively license the programs and undertake further development and potential commercialization.

Fourth Quarter and Full Year 2018 Financial Results and Financial Guidance

Wave reported a net loss of $37.9 million in the fourth quarter of 2018 as compared to $30.8 million in the same period in 2017. The company reported a net loss of $146.7 million for the year ended December 31, 2018 as compared to $102.0 million for the year ended December 31, 2017. The increase in net loss in the fourth quarter and the year ended December 31, 2018 was largely driven by increased research and development efforts and continued organizational growth to support Wave's corporate goals.

Research and development expenses were $39.8 million for the fourth quarter of 2018 as compared to $25.4 million for the same period in 2017. Research and development expenses for the full year were $134.4 million as compared to $79.3 million for the prior year. The increase in research and development expenses in the fourth quarter and full year was primarily due to increases in research, preclinical and clinical activities, further expansion of our manufacturing capabilities and facility-related expenses and related organizational growth to support PRISM.

General and administrative expenses were $12.8 million for the fourth quarter of 2018 as compared to $6.9 million for the same period in the prior year. General and administrative expenses were $39.5 million in 2018 as compared to $27.0 million in 2017. The increase in general and administrative expenses in the fourth quarter and full year was mainly driven by increases in employee headcount to support Wave's corporate goals, as well as increases in professional service expenses and other general operating expenses.

Wave ended 2018 with $174.8 million in cash and cash equivalents as compared to $142.5 million as of December 31, 2017. The increase in cash and cash equivalents was primarily the result of the $170.0 million in cash received from Takeda when the collaboration took effect, which was partially offset by Wave's year-to-date net loss of $146.7 million.

On January 28, 2019, Wave closed a follow-on underwritten public offering of 3,950,000 ordinary shares for gross proceeds of $150.1 million, and on February 26, 2019, Wave closed on the sale of an additional 592,500 ordinary shares pursuant to the underwriters' option (on the same terms and conditions as the initial closing) for gross proceeds of an additional $22.5 million. Net proceeds to Wave from the offering are expected to be approximately $161.6 million, after deducting underwriting discounts and commissions and estimated offering expenses.

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)

	December 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 174,819	$ 142,503
Current portion of accounts receivable	10,000	1,000
Prepaid expenses and other current assets	17,454	6,985
Total current assets	202,273	150,488
Long-term assets:		
Accounts receivable, net of current portion	50,000	—
Property and equipment, net	39,931	27,334
Restricted cash	3,625	3,610
Other assets	111	411
Total long-term assets	93,667	31,355
Total assets	$ 295,940	$ 181,843
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	$ 13,089	$ 7,598
Accrued expenses and other current liabilities	14,736	8,898
Current portion of capital lease obligation	—	16
Current portion of deferred rent	115	60
Current portion of deferred revenue	100,945	1,275
Current portion of lease incentive obligation	1,156	344
Total current liabilities	130,041	18,191
Long-term liabilities:		
Deferred rent, net of current portion	5,132	4,214
Deferred revenue, net of current portion	68,156	7,241
Lease incentive obligation, net of current portion	9,247	3,094
Other liabilities	2,142	1,619
Total long-term liabilities	84,677	16,168
Total liabilities	$ 214,718	$ 34,359
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at December 31, 2018 and 2017	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 29,472,197 and 27,829,079 shares issued and outstanding at December 31, 2018 and 2017, respectively	375,148	310,038
Additional paid-in capital	37,768	22,172
Accumulated other comprehensive income (loss)	153	116
Accumulated deficit	(339,721)	(192,716)
Total shareholders' equity	73,348	139,610
Total liabilities, Series A preferred shares and shareholders' equity	$ 295,940	$ 181,843

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

(In thousands, except share and per share amounts)

	For the Year Ended December 31,		
	2018	2017	2016
Revenue	$ 14,414	$ 3,893	$ 1,092
Operating expenses:			
Research and development	134,428	79,309	40,818
General and administrative	39,509	26,975	15,994
Total operating expenses	173,937	106,284	56,812
Loss from operations	(159,523)	(102,391)	(55,720)
Other income (expense), net:			
Dividend income	3,368	1,578	255
Interest income (expense), net	22	6	337
Other income (expense), net	9,549	(331)	(50)
Other income (expense), net	12,939	1,253	542
Loss before income taxes	(146,584)	(101,138)	(55,178)
Income tax benefit (provision)	(69)	(842)	(482)
Net loss	$ (146,653)	$ (101,980)	$ (55,660)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (5.06)	$ (3.85)	$ (2.44)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	28,970,404	26,513,382	22,800,628
Other comprehensive income (loss):			
Net loss	$ (146,653)	$ (101,980)	$ (55,660)
Foreign currency translation	37	407	(332)
Comprehensive loss	(146,616)	(101,573)	(55,992)

Investor Contact:

Graham Morrell
781-686-9600
gmorrell@wavelifesci.com

Media and Patient Contact:

José Juves
617-949-4708
jjuves@wavelifesci.com

Exhibit 99.2

Wave Life Sciences

Corporate Presentation

March 1, 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.

WAVE™

LIFE SCIENCES

Wave Life Sciences 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.

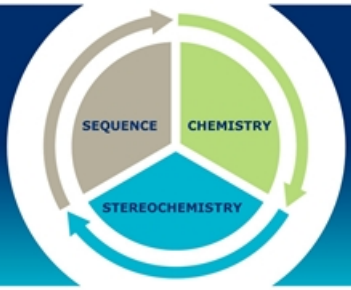
Driven by a resolute sense of urgency, the Wave team is targeting a broad range of genetically defined diseases so that patients and families may realize a brighter future.

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



OPTIMIZATION

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

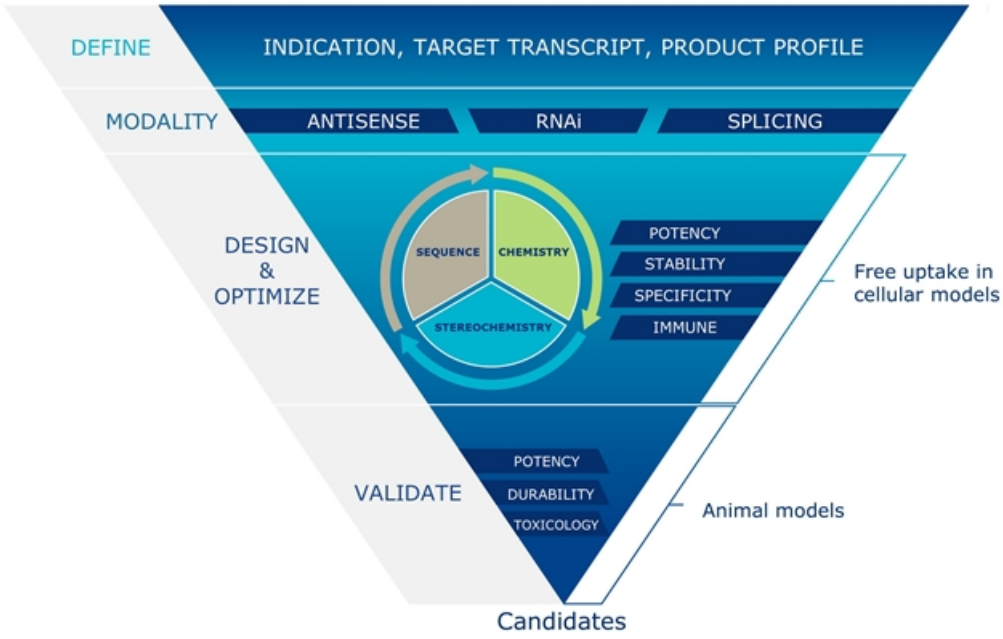
Control of stereochemistry enables the design and manufacture of oligonucleotides with one defined and consistent profile

↓

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

WAVE LIFE SCIENCES

Creating a new class of oligonucleotides

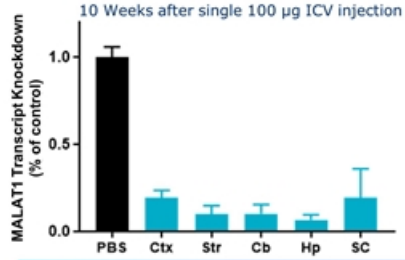


Source: Iwamoto N, et al. Control of phosphorothioate stereochemistry substantially increases the efficacy of antisense oligonucleotides. *Nat Biotechnol*. 2017;35:845-851.

Optimizing potency and durability across multiple tissues

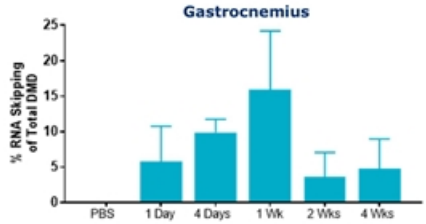
CNS

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



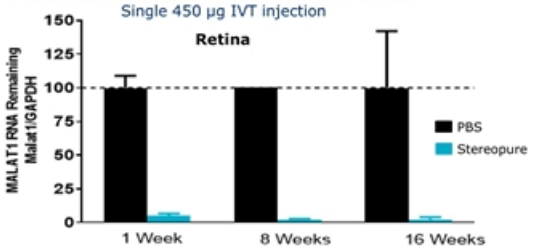
Muscle

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



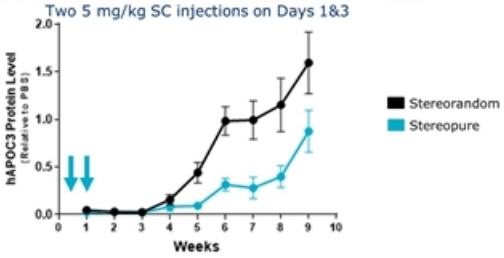
Eye

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

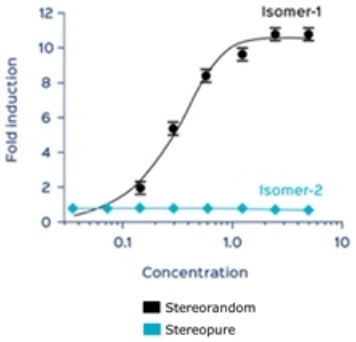


Data represented in this slide from *in vivo* studies. CNS: PBS = phosphate buffered saline; Ctx = cortex; Str = striatum; Cb = cerebellum; Hp = hippocampus; SC = spinal cord. ICV = intracerebral; IVT = intravitreal; IV = intravenous; SC= subcutaneous.

Stereochemistry affects immune activation

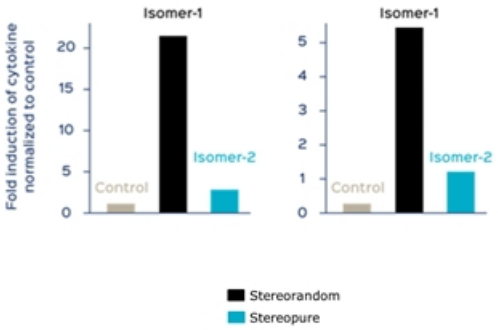
Human TLR9 Activation

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



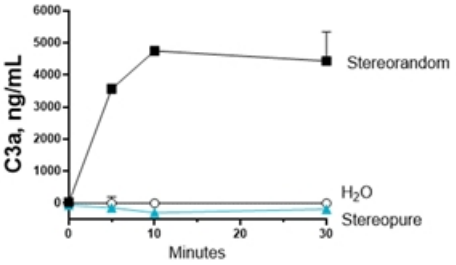
Cytokine Induction

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)	●	●	Phase 1/OLE	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	RHO, USH2A, ABCA4, CEP290	~10,000	○	●	○		100% Global	—
HEPATIC								
Metabolic liver diseases	APOC3 and Multiple (4)‡		(S)	●	○		Milestones & Royalties	Pfizer

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

WAVE™ LIFE SCIENCES

*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.
‡Pfizer has nominated four undisclosed targets in addition to APOC3.

WAVE™
LIFE SCIENCES

Duchenne Muscular Dystrophy (DMD)

DMD: a progressive, fatal childhood disorder

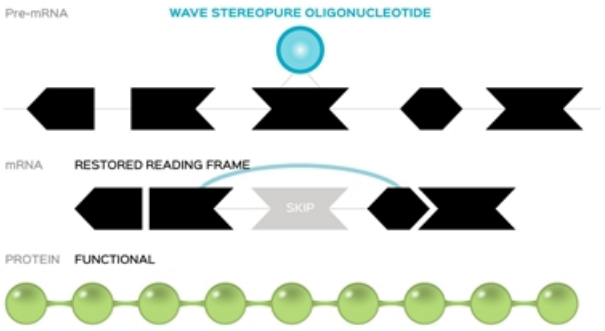
- Fatal, X-linked genetic neuromuscular disorder characterized by progressive, irreversible loss of muscle function, including heart and lung
- Genetic mutation in dystrophin gene prevents the production of dystrophin protein, a critical component of healthy muscle function
- Symptom onset in early childhood; 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

WAVE LIFE SCIENCES

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

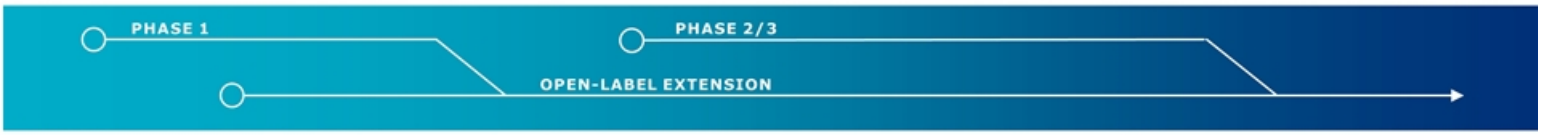
- Chronic administration may better address high muscle cell turnover and need for broad and durable distribution
- Entry into cells, including progenitor cells, via free-uptake
- Production of functional dystrophin protein, not micro-dystrophin
- Scalable manufacturing

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.

WAVE LIFE SCIENCES

Exon 51: suvodirsen (WVE-210201) clinical program



Phase 1

OBJECTIVE

Determine safety and tolerability profile and select dose(s) for OLE and Phase 2/3

STUDY DESCRIPTION

Phase 1 single ascending dose clinical trial

KEY MILESTONES

- Safety and tolerability profile supports Phase 2/3 initiation
- One dose selected for Phase 2/3 trial, pending final analysis
- Results to be presented at upcoming scientific meetings

Open-Label Extension (OLE)

OBJECTIVE

Provide data that will be an important component of submission for accelerated approval in US

STUDY DESCRIPTION

Multi-dose, open-label study open to patients from Phase 1

KEY MILESTONES

- Initiated in August 2018
- On track to deliver interim analysis of dystrophin expression in H2 2019

Phase 2/3

OBJECTIVE

Provide efficacy and safety data as basis of regulatory submissions globally

STUDY DESCRIPTION

Phase 2/3 clinical trial to assess clinical efficacy and dystrophin expression

KEY MILESTONES

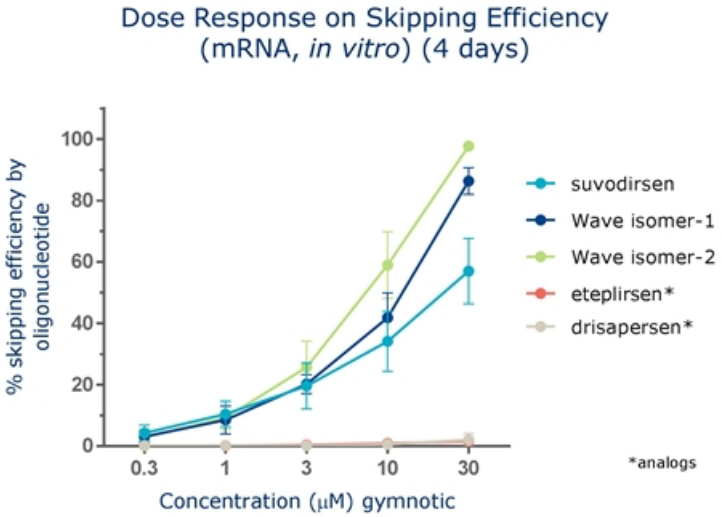
- Selected for FDA pilot program for complex innovative trial designs
- Expect to initiate in 2019

WAVE LIFE SCIENCES

Dystrophin readout expected H2 2019

Exon 51: improved skipping efficiency

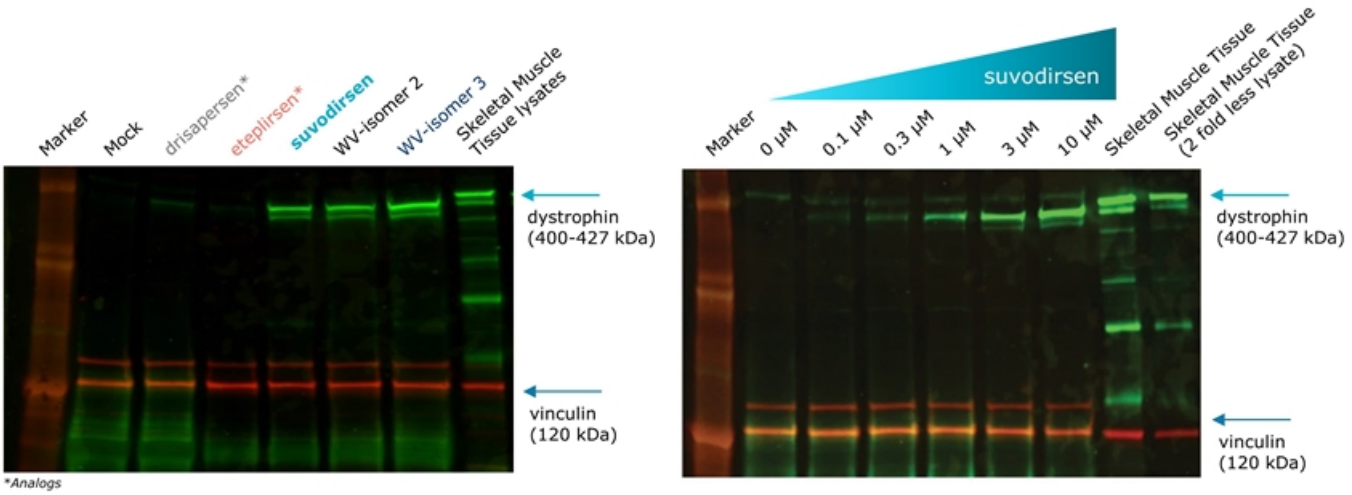
- RNA skipping determined by quantitative RT-PCR
- Wave isomers demonstrated a dose-dependent increase in skipping efficiency *in vitro*
- Free uptake at 10uM concentration of each compound with no transfection agent
- Same foundational stereopure chemistry for Wave isomers; individually optimized to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



WAVE LIFE SCIENCES

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

Exon 51: increased dystrophin restoration



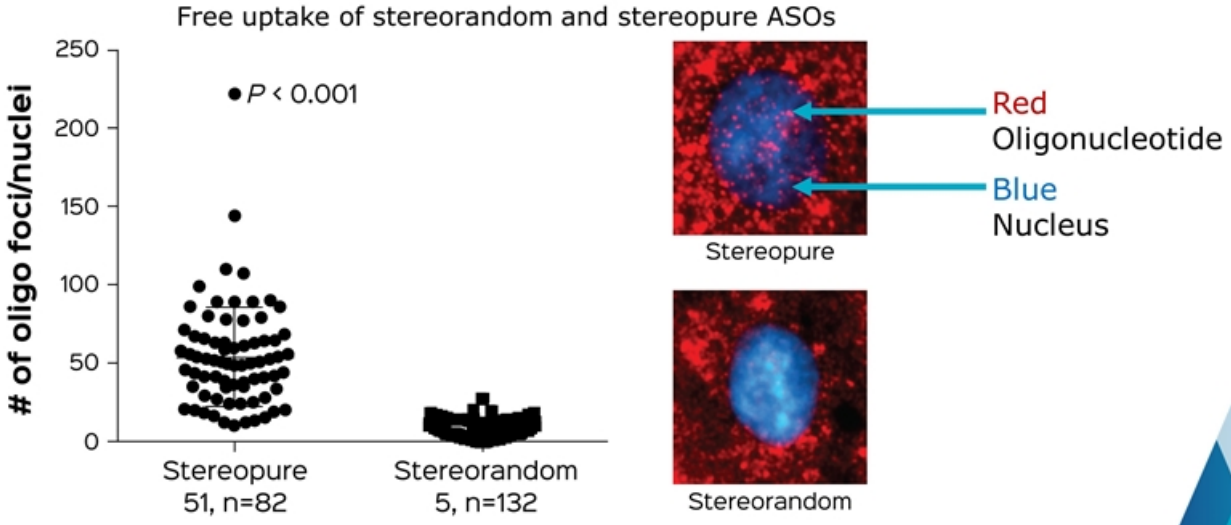
Dystrophin protein restoration *in vitro* was quantified to be between **50-100% of normal** skeletal muscle tissue lysates, as compared to about 1% by drisapersen and eteplirsen analogs

WAVE LIFE SCIENCES

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

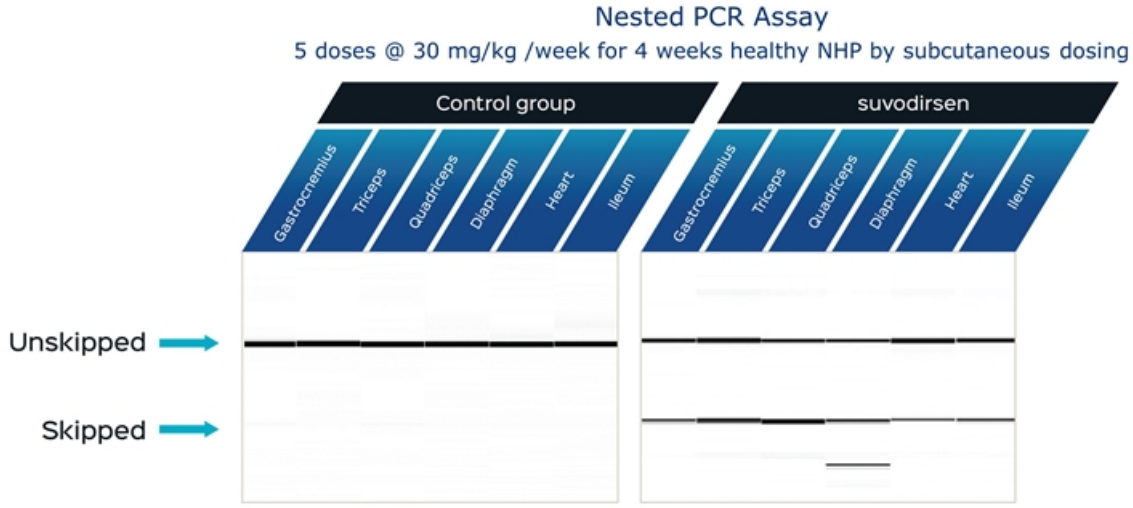
Stereopure oligonucleotides are designed to readily enter the nuclei of cells under free-uptake conditions, which approximates natural delivery in the body



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

WAVE LIFE SCIENCES

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

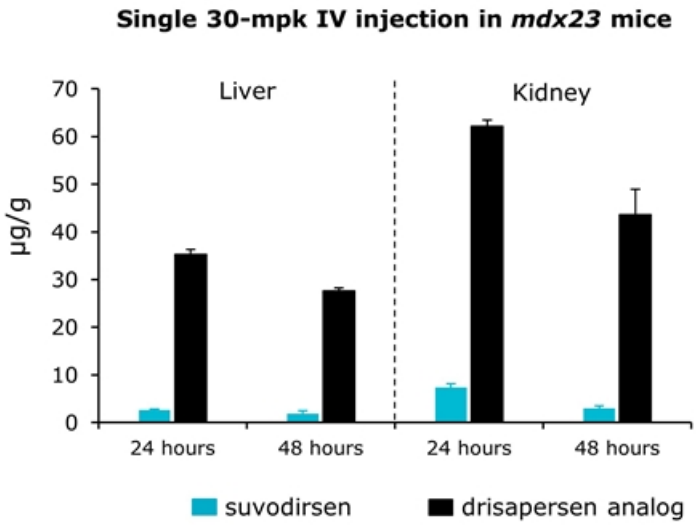


WAVE LIFE SCIENCES

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.

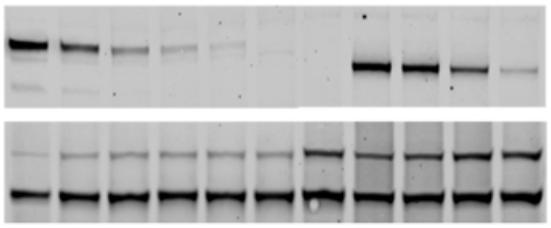
WAVE LIFE SCIENCES

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



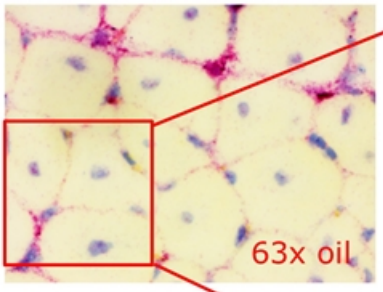
- Free uptake for 6 days in differentiation media with no transfection agent and no peptide conjugated to the oligonucleotide
- Wave stereopure exon 53 candidate demonstrated a dose-dependent increase in dystrophin restoration in DMD patient-derived myoblasts

Topline clinical data expected in H2 2020

WAVE LIFE SCIENCES

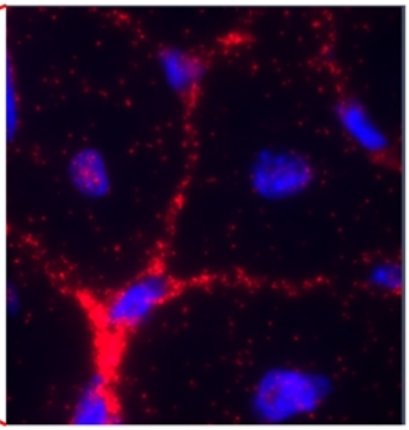
Experimental conditions: D45-52 patient myoblasts were treated with oligonucleotide for 6d under free-uptake conditions in differentiation media. Protein harvested in RIPA buffer and dystrophin restoration analyzed by Western Blot. Signal normalized to vinculin loading control and to primary healthy 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



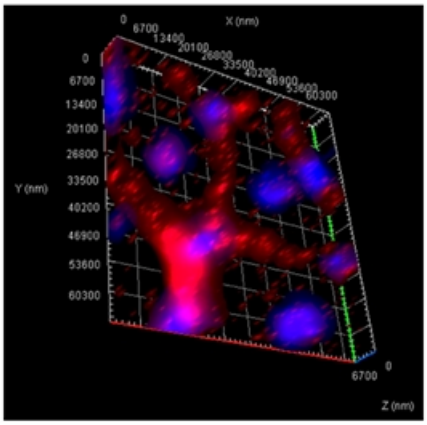
Bright field view

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

WAVE LIFE SCIENCES

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.

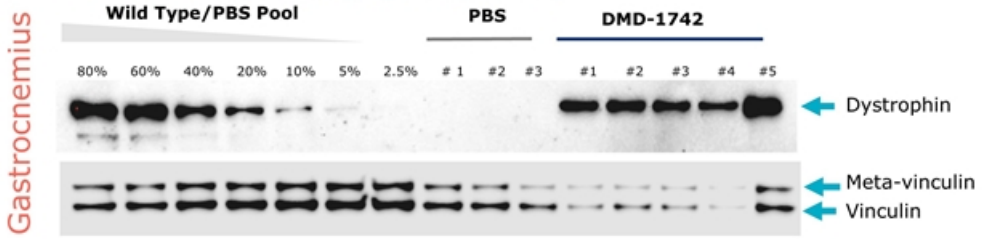
Stereopure surrogate yields substantial dystrophin protein restoration and CK reduction

Multiple Doses (*in vivo mdx23* mice)

Dystrophin Protein Restoration

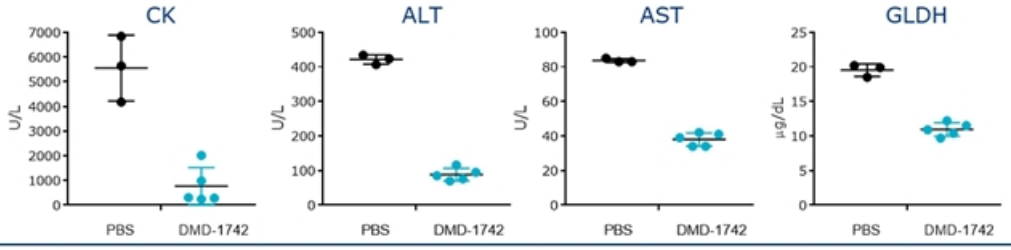
70-90% of dystrophin restoration

DMD-1742 (4 weekly 150-mg/kg IV injections)



Serum Enzyme Levels

87% reduction in creatine kinase (CK) levels



*Numbers indicate individual animals

Note: DMD-1742 is a stereopure oligonucleotide designed to induce exon 23 skipping in the mdx23 mouse model and is a surrogate of suvodirsen, which is designed to induce exon 51 skipping in the human dystrophin transcript

Experimental conditions: Tissues collected 96 hours post final dose. Protein expression determined by Western Blot.

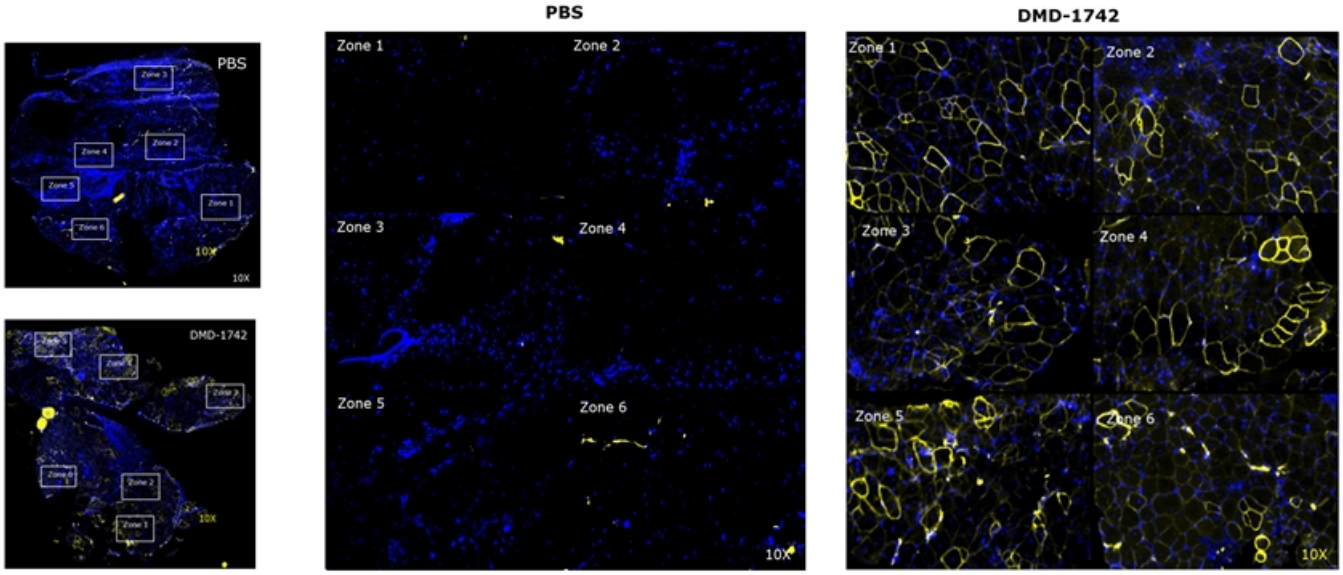
ALT=alanine aminotransferase; AST=aspartate aminotransferase; CK=creatine kinase; GLDH=glutamate dehydrogenase.

Serum and plasma clinical chemistry were measured with an Olympus AU640 (Olympus America) and the manufacturer's reagents and procedures.

WAVE LIFE SCIENCES

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

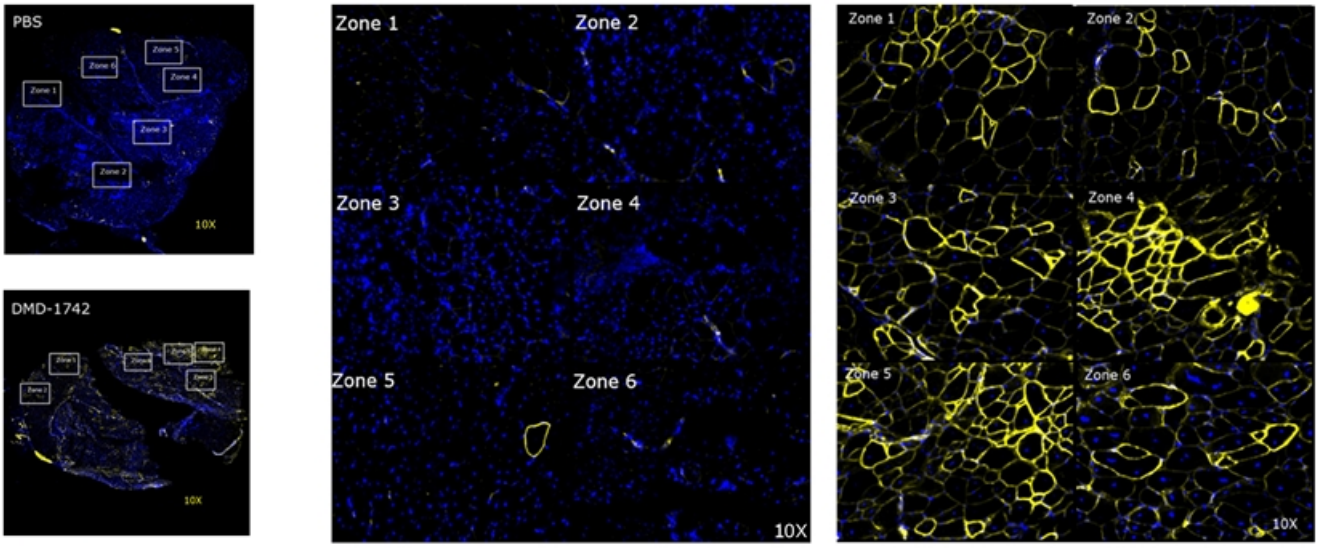
Immunohistochemistry of dystrophin in gastrocnemius in *mdx23* mice at 4 weeks



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

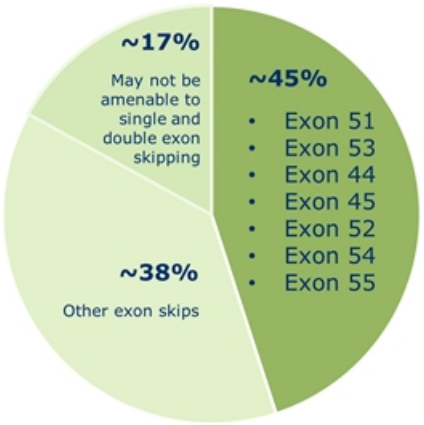


WAVE LIFE SCIENCES

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.

Expansion of stereopure exon skipping DMD portfolio

Percentage of DMD patients amenable to exon skipping therapeutic approach



- Applying learnings from ongoing DMD development efforts and platform advances to explore additional exons for candidate development, including exons 44, 45, 52, 54, 55
- Early leads demonstrate similar *in vitro* exon skipping efficiency as suvodirsen and WVE-N531
- Aim to leverage 21st Century Cures Act to develop additional candidates

Committed to unlocking the promise of genetic medicines to advance the treatment of DMD

WAVE LIFE SCIENCES

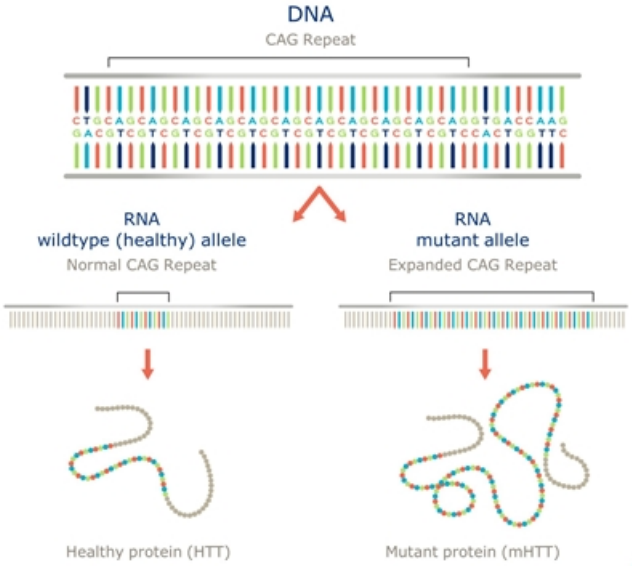
Sources: Aartsma-Rus A, et al. *Hum Mutat.* 2009;30:293-299. Bladen CL, et al. *Hum Mutat.* 2015;36:395–402.

WAVE™
LIFE SCIENCES

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

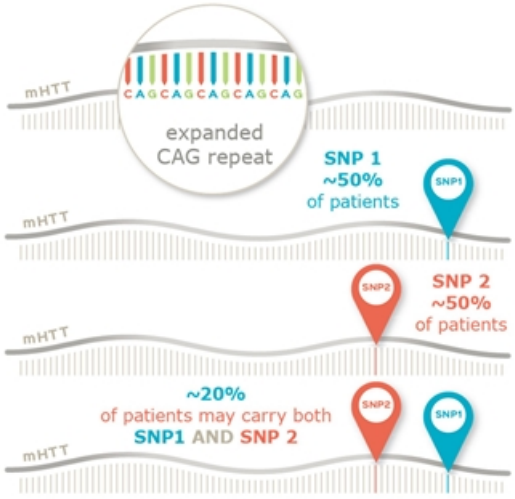


Sources: Auerbach W, et al. *Hum Mol Genet*. 2001;10:2515-2523. Dragatsis I, et al. *Nat Genet*. 2000;26:300-306. Leavitt BR, et al. *J Neurochem*. 2006;96:1121-1129. Nasir J, et al. *Cell*. 1995;81:811-823. Reiner A, et al. *J Neurosci*. 2001;21:7608-7619. White JK, et al. *Nat Genet*. 1997;17:404-410. 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 LIFE SCIENCES

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

WAVE LIFE SCIENCES

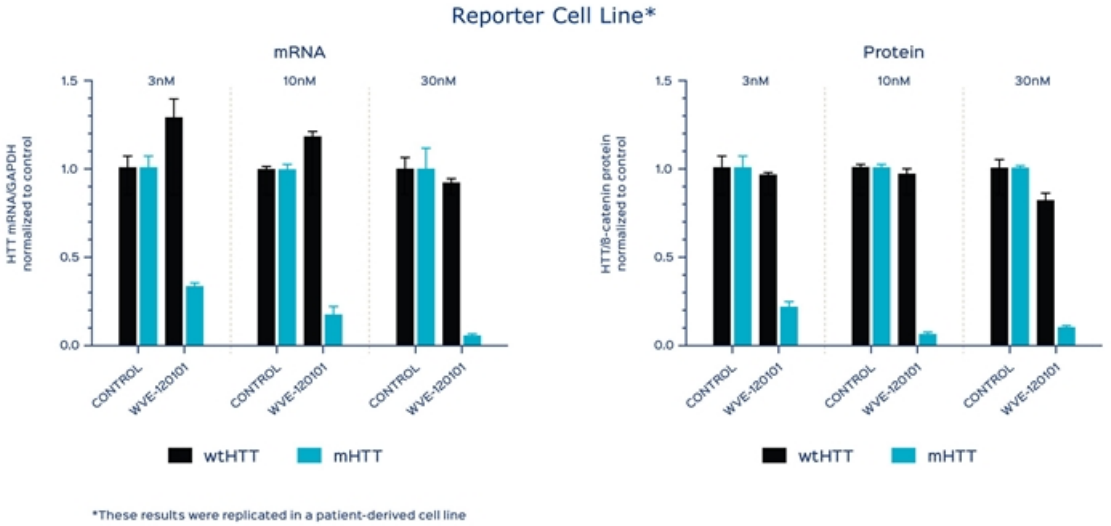
Source: Kay, et al. Personalized gene silencing therapeutics for Huntington disease. *Clin Genet.* 2014;86:29–36.

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 H1 2019

Selective reduction of mHTT mRNA & protein

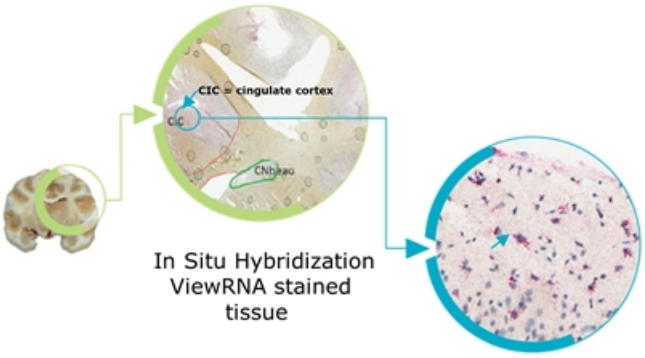


*These results were replicated in a patient-derived cell line

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

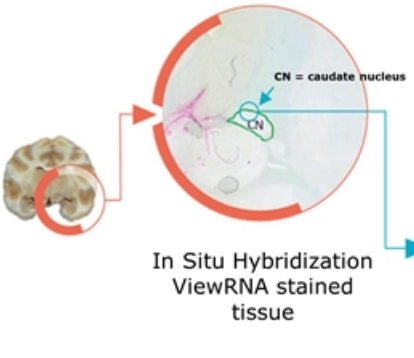
- WVE-120101 and WVE-120102 distribution in cynomolgus non-human primate brain following intrathecal bolus injection



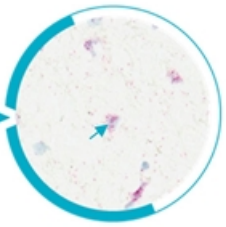
In Situ Hybridization ViewRNA stained tissue

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

WAVE LIFE SCIENCES

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.

WAVE™
LIFE SCIENCES

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

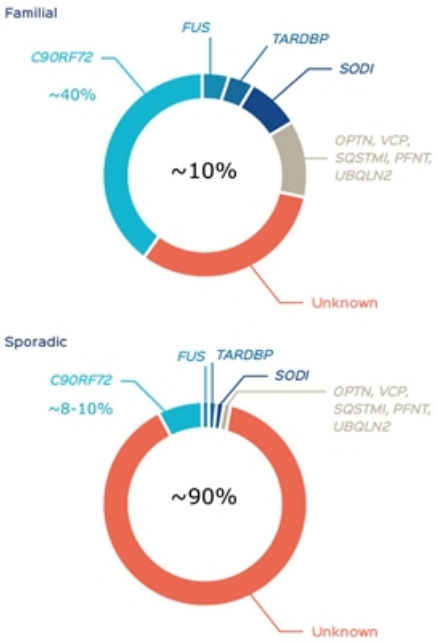
WAVE LIFE SCIENCES

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

Topline clinical data expected in H2 2020

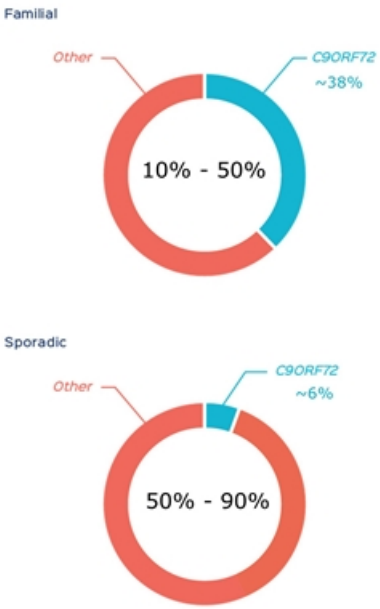


WAVE LIFE SCIENCES

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

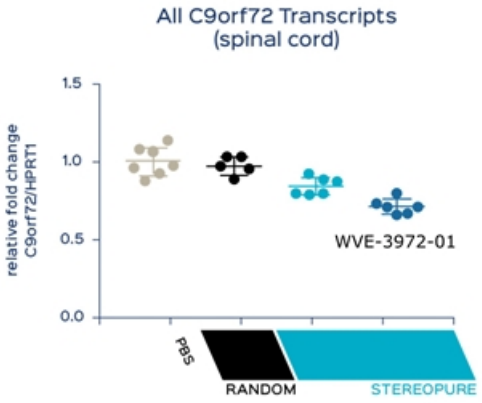


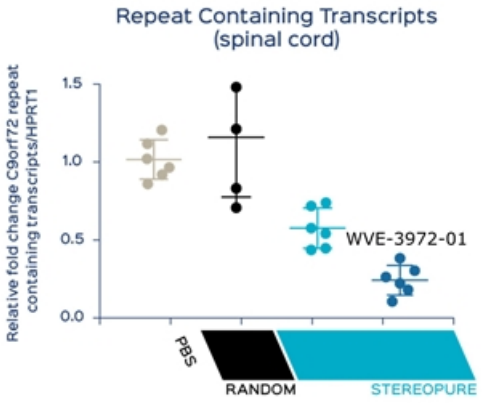
Topline clinical data expected in H2 2020

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.

Selective silencing *in vivo* of expanded C9orf72 repeat transcripts

- Wave has developed a series of highly optimized antisense compounds which selectively silence the repeat containing transcript in C9orf72 transgenic mice
- These compounds show target engagement across cell types and regions of the nervous system critically implicated in ALS and FTD



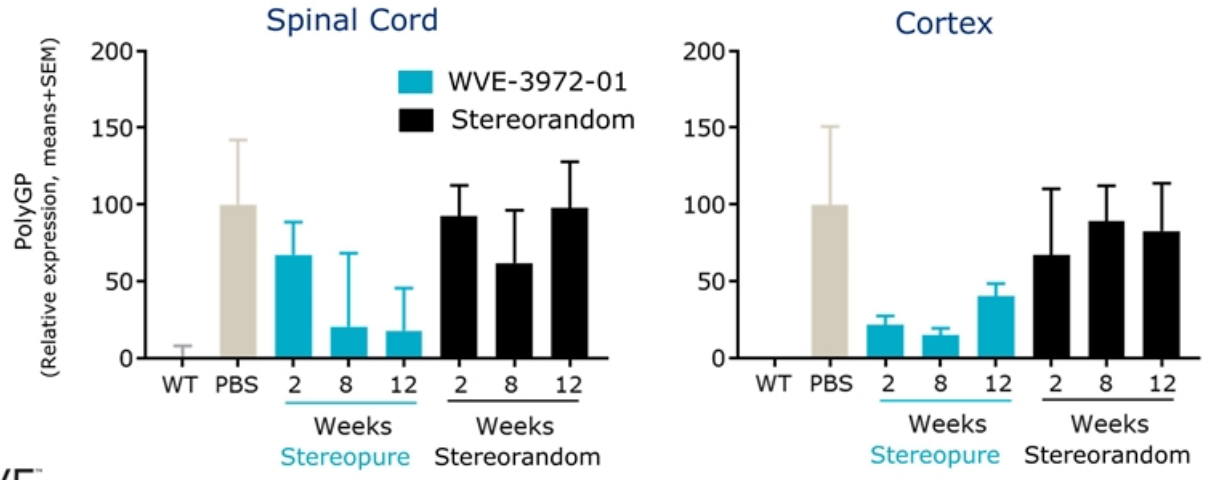


WAVE LIFE SCIENCES

Experimental description: Samples were analyzed using quantitative PCR (Taqman assay)

WVE-3972-01 produces durable reduction in dipeptides *in vivo*

Durable reduction of dipeptide in spinal cord and cortex in C9-BAC mice for at least 12 weeks

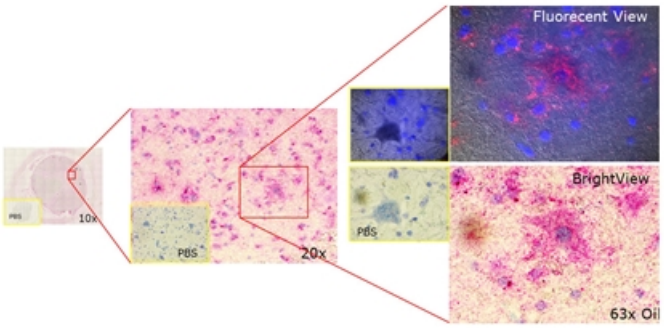


WAVE LIFE SCIENCES

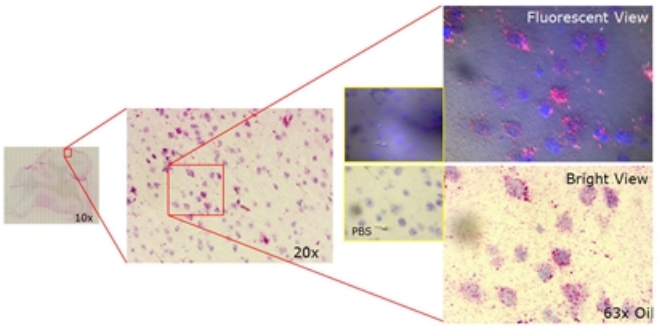
Experimental design: C9-BAC mice received a single ICV injection of PBS or oligonucleotide (100 μg).

WVE-3972-01 in nuclei of neurons in NHP CNS

Spinal cord: Oligonucleotide visualization (ViewRNA) after intrathecal delivery in NHPs



Frontal Cortex: Oligonucleotide visualization (ViewRNA) after intrathecal delivery in NHPs



Blue: Nuclear, Hematoxylin; Pink Red: ASO/ViewRNA, Fast Red/Cy3

Widespread and sustained distribution in nuclei of neurons in spinal cord and frontal cortex

NHP = non-human primate.
Experimental design: Cynomolgus monkeys were administered 3 weekly IT doses of ASO; tissues were collected 48 hours after last injection.

WAVE™
LIFE SCIENCES

Ophthalmology

Building a portfolio for inherited retinal diseases

Inherited retinal diseases (IRDs)

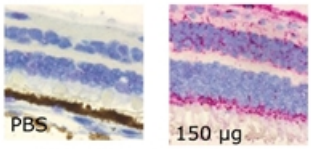
- Rare eye disorders caused by genetic mutations leading to progressive vision loss
- No approved therapies for almost any IRDs
- Approximately 200,000 affected in the U.S. and millions world-wide

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

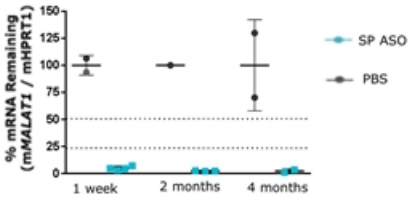
Single IVT injection of stereopure oligonucleotide to NHP results in distribution throughout all layers of the retina and potent, extended duration of effect

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 H2 2019

WAVE LIFE SCIENCES

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.

WAVE™
LIFE SCIENCES

Partnerships

Collaborating to maximize portfolio and platform

Takeda

- **$230+ million** in committed cash; eligible for milestones and royalties in excess of $2 billion*
- Takeda option on **global 50:50 share** of CNS programs in HD, ALS, FTD and SCA3
- **Fully funded CNS R&D** with Takeda right to license additional preclinical CNS targets over four years

Pfizer

- **$40 million** upfront payment; **$871 million** in potential milestone payments and royalties
- **Advancing 5 targets**, including APOC3, for the treatment of metabolic liver diseases
- Leveraging **Wave proprietary chemistry platform** across modalities with GalNAc and Pfizer's hepatic targeting technology

Platform technologies

deep genomics

- Applying **artificial intelligence** to discover novel therapies for genetic neuromuscular disorders

READCOOR

- Utilizing **3D imaging** to assess target engagement in specific regions, cell types and subcellular compartments of the brain

WAVE LIFE SCIENCES

*Assuming Takeda advances six programs that achieve regulatory approval and commercial sales, Wave will be eligible to receive up to $2 billion in cash milestone payments, of which more than $1 billion would be in precommercial milestone payments.

Upcoming Wave catalysts

- **H1 2019: Data expected in HD from Phase 1b/2a trials for WVE-120101 and WVE-120102**
  - Potential to be first two allele-specific disease-modifying therapies selectively lowering mHTT
- **2019: Initiate Phase 2/3 clinical trial for suvodirsen (WVE-210201) in DMD**
  - Protocol selected for FDA complex innovative trial designs (CID) pilot program
- **H2 2019: Interim dystrophin data readout expected in DMD for suvodirsen (WVE-210201)**
- **H2 2019: Initial development candidate for inherited retinal disease**
- **H2 2020:**
  - Anticipate filing an NDA and pursuing accelerated approval for suvodirsen (WVE-210201) in exon 51 amenable DMD
  - Topline clinical data expected in DMD for WVE-N531 targeting exon 53
  - Topline clinical data expected from WVE-3972-01 C9orf72 programs

WAVE LIFE SCIENCES

WAVE™
LIFE SCIENCES

Realizing the potential of genetic medicines

For more information:
Graham Morrell, Investor Relations
gmorrell@wavelifesci.com
781.686.9600