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 2, 2020

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

Singapore	**001-37627**	**00-0000000**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

7 Straits View #12-00, Marina One East Tower
Singapore **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	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 March 2, 2020, Wave Life Sciences Ltd. (the "Company") announced its financial results for the quarter and year ended December 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 March 2, 2020, 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 March 2, 2020
99.2	Corporate Presentation of Wave Life Sciences Ltd. dated March 2, 2020
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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/ Paul B. Bolno, M.D.*
Paul B. Bolno, M.D.
President and Chief Executive Officer

Date: March 2, 2020

Wave Life Sciences Reports Fourth Quarter and Full Year 2019 Financial Results and Provides Business Update

Innovative pipeline of stereopure oligonucleotides focused on CNS diseases

32 mg data from both PRECISION-HD clinical trials on track for 2H 2020

Two additional CNS programs – SNP3 and C9orf72 – on track to initiate clinical development in 2H 2020

Wave to host investor conference call and webcast at 8:00 a.m. ET today

CAMBRIDGE, Mass., March 2, 2020 – 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 fourth quarter and full year ended December 31, 2019 and provided a business update.

"We continue to advance our innovative, CNS-focused pipeline of stereopure oligonucleotides across Huntington's disease, amyotrophic lateral sclerosis, frontotemporal dementia and other central nervous system diseases. Despite the disappointment of discontinuing our Duchenne program last year, we are advancing more than a dozen programs across discovery and development with several exciting milestones ahead in 2020," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "In December 2019, we shared the first clinical results from an allele-selective program for Huntington's disease. Initial results from PRECISION-HD2 demonstrated a reduction in mutant huntingtin protein, with a safety profile that supports the addition of a higher dose cohort, and no change in total huntingtin protein as compared to placebo. In the second half of 2020, we expect to initiate clinical development for our SNP3 program as well as our C9orf72 program, both of which have benefitted from novel advances in our PRISM platform. In addition, we are pleased by recent progress across multiple CNS programs we are working on in collaboration with Takeda. Finally, we presented proof-of-concept data for our RNA-editing program in January, which demonstrated endogenous ADAR engagement *in vitro*. We expect to have initial *in vivo* results in 2020, and we look forward to sharing further updates on this exciting new modality."

Business update

Wave is building a leading genetic medicines company focused on realizing the potential of stereopure oligonucleotides in diseases of the central nervous system, liver, and eye. Wave's pipeline includes more than a dozen programs across discovery and development, spans multiple modalities and targets, and is intended to deliver transformational medicines to patients and families.

Central nervous system (CNS) diseases

Updates for PRECISION-HD clinical trials of WVE-120101 and WVE-120102 in Huntington's disease

- *WVE-120101 and WVE-120102 allele-selectivity*: Investigational WVE-120101 and WVE-120102 are currently the only compounds in clinical development designed to selectively target the mutant allele of the huntingtin (mHTT) gene, while leaving the wild-type (wtHTT) relatively intact. The wtHTT protein is important for neuronal function, and there is increasing evidence that it may be neuroprotective in an adult brain. Additionally, Huntington's disease (HD) may be caused by a dominant gain of function in mHTT protein *and* a concurrent loss

of function of wtHTT protein may be an important component of the pathophysiology of HD. Wave's allele-selective approach may also enable the company to address the pre-manifest, or asymptomatic, HD patient population in the future.

- *PRECISION-HD2*: In January 2020, Wave initiated dosing in a 32 milligram (mg) dose cohort in the PRECISION-HD2 Phase 1b/2a clinical trial of WVE-120102, a stereopure oligonucleotide designed to selectively target the mutant huntingtin (mHTT) mRNA transcript of SNP2 for HD. Wave is also assessing the potential for a next higher dose cohort to be added to the trial.
- Wave's ability to advance to higher dose cohorts was supported by initial PRECISION-HD2 clinical data, announced in December 2019, that demonstrated a statistically significant reduction of 12.4% in mHTT in cerebrospinal fluid (CSF) in an analysis comparing all patients treated with WVE-120102 to placebo. An analysis to assess a dose response across treatment groups (2, 4, 8 or 16 mg) suggested a statistically significant response in mHTT reduction at the highest doses tested (p=0.03). WVE-120102 was generally safe and well tolerated across all cohorts. There was no difference in total huntingtin protein compared to placebo.
- Data from the 32 mg dose cohort of the PRECISION-HD2 trial are expected in the second half of 2020.
- Enrollment continues in an open-label extension (OLE) study open to patients outside of the U.S. who participated in the Phase 1b/2a PRECISION-HD2 trial.
- *PRECISION-HD1*: The PRECISION-HD1 Phase 1b/2a clinical trial, Wave's clinical trial investigating WVE-120101, a stereopure oligonucleotide designed to selectively target the mHTT mRNA transcript of SNP1 for HD, is ongoing. Wave expects to initiate a 32 mg cohort to the PRECISION-HD1 trial and deliver topline clinical data from the PRECISION-HD1 trial, including a 32 mg dose cohort, in the second half of 2020.
- In February 2020, Wave initiated an OLE study open to patients outside of the U.S. who participated in the Phase 1b/2a PRECISION-HD1 trial.

Advancing third allele-selective Huntington's disease program (SNP3) towards clinical development.

- In February 2020, at the 15th annual CHDI Foundation Huntington's Disease Therapeutics Conference, Wave presented preclinical data for its investigational SNP3 program. SNP3 represents ~40% of the HD population and, with overlap, up to 80% of the HD population carries at least one of SNP1, SNP2, and/or SNP3. In patient-derived neurons, Wave's allele-selective SNP3 compounds demonstrated more potent knockdown of mutant HTT in vitro than a pan-silencing active comparator. In addition, Wave's SNP3 compounds demonstrated potent and durable knockdown of mutant HTT in a transgenic mouse model for up to 12 weeks.
- Wave expects to initiate clinical development of its SNP3 program in the second half of 2020.

Advancing C9orf72 preclinical program for ALS and FTD towards clinical development

- Wave is advancing its C9orf72 preclinical program to potentially treat amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) and expects to initiate clinical development in the second half of 2020.Wave's C9orf72 program preferentially targets the transcript containing the hexanucleotide repeat expansion (G4C2) in the *C9orf72* gene.
- Wave's C9orf72 program led to substantial reduction of repeat containing *C9orf72* transcripts and dipeptides in both the spinal cord and cortex of a transgenic mouse model, while total C9orf72 protein was preserved.

Pipeline of CNS programs progressing in collaboration with Takeda

- The company is leveraging its learnings from PRISM™ to design additional stereopure oligonucleotides with optimized profiles for CNS indications, including Parkinson's, Alzheimer's and others as part of its ongoing collaboration with Takeda.

Ophthalmologic diseases

- In 2019, Wave's discovery efforts yielded two preclinical ophthalmology programs. The first program uses stereopure oligonucleotides to promote *USH2A* exon 13 skipping to address Usher Syndrome Type 2A. The second program uses stereopure oligonucleotides to selectively silence RhoP23H transcripts to address retinitis pigmentosa.

RNA-editing

- Wave is leveraging its proprietary PRISM platform to design novel RNA-editing therapeutics. Wave's technology uses endogenous ADAR (adenosine deaminases acting on RNA) enzymes via non-viral, free uptake of RNA editing oligonucleotides in a variety of primary human cell types *in vitro* with high efficiencies and has potential to be a best-in-class RNA editing modality.
- In January 2020, at the 1st International Conference on Base Editing – Enzymes and Applications (Deaminet 2020), Wave presented a poster titled "RNA Editing via Endogenous ADARs Using Stereopure Oligonucleotides." Wave observed preclinical editing efficiencies of up to 70% in primary hepatocytes and approximately 50% in bronchial epithelial cells without the need for viral or lipid nanoparticle (LNP) delivery vehicles. In addition, stereopure oligonucleotides achieved greater ADAR-mediated editing compared to stereorandom oligonucleotides across several distinct RNA transcripts in primary human hepatocytes, which validates that the technology is applicable across multiple sequences.
- Wave expects to share *in vivo* RNA editing data in 2020.

Neuromuscular disease

- In December 2019, Wave announced the discontinuation of suvodirsen development for patients with Duchenne muscular dystrophy (DMD). Wave plans to present additional findings from the Phase 1 open-label extension study of suvodirsen at the 2020 MDA Clinical and Scientific Conference in Orlando, Florida, which will take place from March 20 through March 25, 2020.

Fourth Quarter and Full Year 2019 Financial Results and Financial Guidance

Wave reported a net loss of $56.8 million in the fourth quarter of 2019 as compared to $37.9 million in the same period in 2018. The company reported a net loss of $193.6 million for the year ended December 31, 2019 as compared to $146.7 million for the year ended December 31, 2018. The increase in net loss in the fourth quarter and the year ended December 31, 2019 was largely driven by increased research and development efforts and continued organizational growth, both of which included costs and efforts, including manufacturing, in preparation for the potential commercialization of suvodirsen.

Research and development expenses were $49.1 million in the fourth quarter of 2019 as compared to $39.8 million in the same period in 2018. Research and development expenses for the full year were $175.4 million as compared to $134.4 million for the prior year. The increase in research and development expenses in the fourth quarter and full year was primarily due to increased external expenses related to our clinical activities, including our HD programs and our now discontinued DMD programs, as well as increased investments in PRISM and other research and development expenses.

General and administrative expenses were $13.8 million for the fourth quarter of 2019 as compared to $12.8 million for the same period in the prior year. General and administrative expenses were $48.9 million in 2019 as compared to $39.5 million in 2018. The increase in general and administrative expenses in the fourth quarter and full year was mainly driven by continued organizational growth to support Wave's 2019 corporate goals.

Wave ended 2019 with $147.2 million in cash and cash equivalents as compared to $174.8 million as of December 31, 2018. The decrease in cash and cash equivalents was primarily the result of Wave's year-to-date net loss of $193.6 million, partially offset by the $161.8 million in net proceeds from the January 2019 follow-on offering.

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 into the third quarter of 2021.

Investor Conference Call and Webcast

Wave management will host an investor conference call today at 8:00 a.m. ET to discuss the company's fourth quarter and full year 2019 operating results and provide an update on the company's development programs. The conference call may be accessed by dialing (866) 220-8068 (domestic) or +1 (470) 495-9153 (international) and entering conference ID 3994836. The live webcast may be accessed by visiting the investor relations section of the Wave Life Sciences corporate website at www.ir.wavelifesciences.com. Following the webcast, a replay will be available on the website.

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; regulatory submissions; 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 capabilities; 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 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 interactions; 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 our 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 indications, 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, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 147,161	$ 174,819
Current portion of accounts receivable	20,000	10,000
Prepaid expenses	9,626	6,587
Other current assets	8,689	10,867
Total current assets	185,476	202,273
Long-term assets:		
Accounts receivable, net of current portion	30,000	50,000
Property and equipment, net	36,368	39,931
Operating lease right-of-use assets	18,101	—
Restricted cash	3,647	3,625
Other assets	10,658	111
Total long-term assets	98,774	93,667
Total assets	$ 284,250	$ 295,940
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	$ 9,073	$ 13,089
Accrued expenses and other current liabilities	16,185	14,736
Current portion of deferred rent	—	115
Current portion of deferred revenue	89,652	100,945
Current portion of lease incentive obligation	—	1,156
Current portion of operating lease liability	3,243	—
Total current liabilities	118,153	130,041
Long-term liabilities:		
Deferred rent, net of current portion	—	5,132
Deferred revenue, net of current portion	63,466	68,156
Lease incentive obligation, net of current portion	—	9,247
Operating lease liability, net of current portion	29,304	—
Other liabilities	1,721	2,142
Total long-term liabilities	94,491	84,677
Total liabilities	$ 212,644	$ 214,718
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at December 31, 2019 and 2018	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 34,340,690 and 29,472,197 shares issued and outstanding at December 31, 2019 and 2018, respectively	539,547	375,148
Additional paid-in capital	57,277	37,768
Accumulated other comprehensive income	267	153
Accumulated deficit	(533,359)	(339,721)
Total shareholders' equity	63,732	73,348
Total liabilities, Series A preferred shares and shareholders' equity	$ 284,250	$ 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 December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
Revenue	$ 2,400	$ 3,620	$ 15,983	$ 14,414
Operating expenses:				
Research and development	49,128	39,809	175,431	134,428
General and administrative	13,805	12,754	48,869	39,509
Total operating expenses	62,933	52,563	224,300	173,937
Loss from operations	(60,533)	(48,943)	(208,317)	(159,523)
Other income, net:				
Dividend income	736	1,014	4,912	3,368
Interest income, net	4	6	29	22
Other income (expense), net	3,023	9,933	9,738	9,549
Total other income, net	3,763	10,953	14,679	12,939
Loss before income taxes	(56,770)	(37,990)	(193,638)	(146,584)
Income tax provision	—	103	—	(69)
Net loss	$ (56,770)	$ (37,887)	$ (193,638)	$ (146,653)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (1.65)	$ (1.29)	$ (5.72)	$ (5.06)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	34,303,975	29,463,131	33,866,487	28,970,404
Other comprehensive income (loss):				
Net loss	$ (56,770)	$ (37,887)	$ (193,638)	$ (146,653)
Foreign currency translation	(15)	(28)	114	37
Comprehensive loss	$ (56,785)	$ (37,915)	$ (193,524)	$ (146,616)

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Exhibit 99.2

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Wave Life Sciences

Corporate Presentation

March 2, 2020

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.

Building a leading genetic medicines company

INNOVATIVE PLATFORM

- Stereopure oligonucleotides
- Backbone modifications
- Allele-selectivity
- Novel modalities (ADAR)
- Foundational stereochemistry IP

FOUNDATION OF CNS PROGRAMS

- Huntington's disease
- ALS / FTD
- Ataxias
- Parkinson's
- Alzheimer's

PRISM

CLINICAL DEVELOPMENT EXPERTISE

- Multiple global clinical trials ongoing across eight countries
- Innovative trial designs

MANUFACTURING

- Established internal manufacturing capabilities to produce oligonucleotides at scale

Innovative pipeline led by CNS programs

THERAPEUTIC AREA	TARGET	DISCOVERY	PRECLINICAL	CLINICAL	ESTIMATED U.S. PREVALENCE*	PARTNER
CNS						
Huntington's disease	**WVE-120101** mHTT SNP1			Phase 1b/2a and OLE	~10,000 / ~35,000	Takeda 50:50 option
	WVE-120102 mHTT SNP2			Phase 1b/2a and OLE	~10,000 / ~35,000	Takeda 50:50 option
	mHTT SNP3				~8,000 / ~30,000	Takeda 50:50 option
ALS and FTD	C9orf72				~1,800 (ALS) ~7,000 (FTD)	Takeda 50:50 option
Spinocerebellar ataxia 3	ATXN3				~4,500	Takeda 50:50 option
CNS diseases	Multiple†					Takeda milestones & royalties
OPHTHALMOLOGY						
Retinal diseases	USH2A and RhoP23H					100% global
HEPATIC						
Metabolic liver diseases	Multiple					Pfizer milestones & royalties
OTHER						
ADAR RNA-editing	Multiple					100% global

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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.
ALS: Amyotrophic lateral sclerosis; FTD: Frontotemporal dementia; CNS: Central nervous system; OLE: Open-label extension

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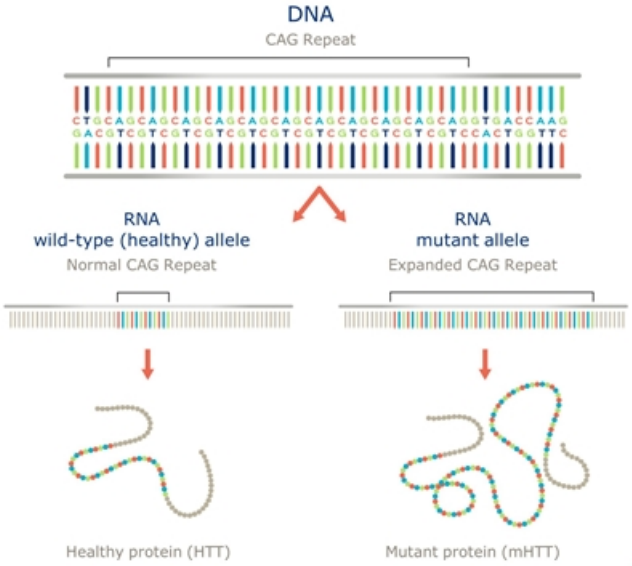
HD portfolio
Huntington's Disease

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
- Wild-type (healthy) HTT protein critical for neuronal function; evidence suggests wild-type HTT loss of function plays a role in Huntington's disease
- 30,000 people with Huntington's disease in the US; another 200,000 at risk of developing the condition



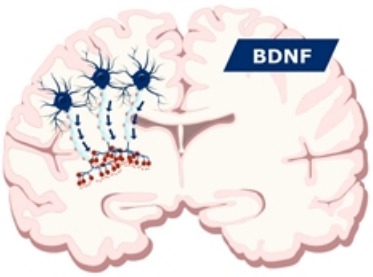
Sources: Auerbach W, et al. *Hum Mol Genet.* 2001;10:2515-2523. Dragatsis I, et al. *Nat Genet.* 2000;26:300-306. Leavitt BR, et al. *J Neurochem.* 2006;96:1121-1129. Nasir J, et al. *Cell.* 1995;81:811-823. Reiner A, et al. *J Neurosci.* 2001;21:7608-7619. White JK, et al. *Nat Genet.* 1997;17:404-410. Zeitlin S, et al. *Nat Genet.* 1995;11:155-163. Carroll JB, et al. *Mol Ther.* 2011;19:2178-2185. HDSA 'What is Huntington's disease?' https://hdsa.org/what-is-hd/overview-of-huntingtons-disease/ Accessed: 11/2/18.; Becanovic, K., et al., Nat Neurosci, 2015. 18(6): p. 807-16. Van Raamsdonk, J.M., et al., Hum Mol Genet, 2005. 14(10): p. 1379-92.; Van Raamsdonk, J.M., et al., BMC Neurosci, 2006. 7: p. 80.

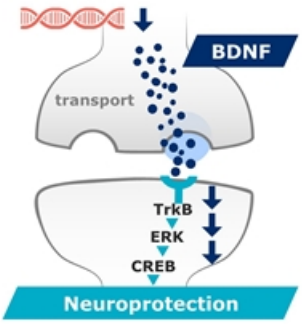
WAVE LIFE SCIENCES

Importance of wild-type huntingtin (wtHTT) in HD

Huntington's disease (HD) may be caused by a dominant gain of function in mutant HTT *and* a loss of function of wtHTT protein

- Evidence suggests wild-type or healthy HTT is neuroprotective in an adult brain
  - Transport of key neurotrophic factors such as brain-derived neurotrophic factor (BDNF) are regulated by wtHTT levels
- Relative proportion of wild-type to mutant protein is critical
  - Increased amount of wild-type protein relative to mutant HTT may result in slower disease progression (measured by age-at-onset)
  - Patients with lack of wild-type have significantly more severe disease (measured by disease progression after symptom onset)





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Sources: Van Raamsdonk, J.M., et al., Hum Mol Genet, 2005; Van Raamsdonk, J.M., et al., BMC Neurosci, 2006; Becanovic, K., et al., Nat Neurosci, 2015; Saudou, F. and S. Humbert, The Biology of Huntingtin. Neuron, 2016; Gauthier, L.R., et al., Cell, 2004; Caviston, J.P. and E.L. Holzbaur, Trends Cell Biol, 2009; Ho, L.W., et al., J Med Genet, 2001, Zuccato et al., Science 2001; Zuccato et al., Brain Pathol 2007; Marullo et al. Genome Biol 2010; Squitieri et. al, Brain 2003

Increasing evidence on the importance of wtHTT in HD pathogenesis, CNS and systemic health

Recent publications on wtHTT LoF as a likely driver of HD pathogenesis

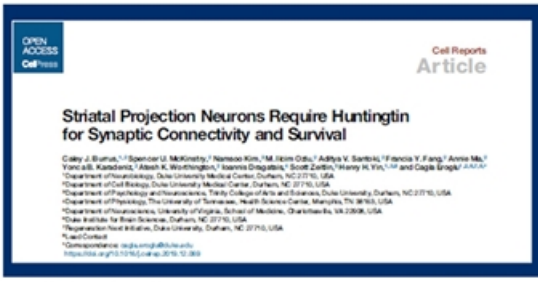

Neurobiology of Disease

journal homepage: www.elsevier.com/locate/ynbdi

Loss of huntingtin function slows synaptic vesicle endocytosis in striatal neurons from the $htt^{Q140/Q140}$ mouse model of Huntington's disease

Robyn L. McAdam[1], Andrew Morton[2,3], Sarah L. Gordon[2,3], Julia F. Alterman[2,*], Anastasia Khvorova[2,*], Michael A. Cousin[4,*], Karen J. Smillie[4,*]

- Striatum-specific defect in synaptic vesicle endocytosis that was not corrected by total lowering of HTT
- Corrected by overexpression of wild-type protein


OPEN ACCESS

Cell Reports

Article

Striatal Projection Neurons Require Huntingtin for Synaptic Connectivity and Survival

- Striatal projection neurons require HTT for motor regulation, synaptic development, cell health, and survival during aging
- Loss of HTT function could play a critical role in HD pathogenesis

wtHTT in HD highlighted at CHDI 15th Annual HD Therapeutics Conference:

HTT LOWERING: EXPLORING DISTRIBUTION, TIMING, AND SAFETY (LOSS OF FUNCTION)

Key points discussed at meeting:

- wtHTT has numerous critical functions throughout life (e.g., intracellular trafficking, cell-cell adhesion, BDNF transport)
- Near elimination of mouse wtHtt detrimental regardless of when suppression begins
- Specific brain regions, e.g., STN, may be particularly vulnerable to wtHTT lowering
- Mouse Htt lowering can lead to thalamic, hepatic, pancreatic toxicity
- HTT LoF mutations highly constrained in human population, suggesting selection against LoF mutations

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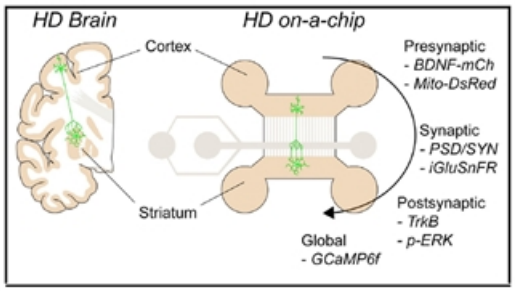
LoF: Loss of function; wtHTT: wild-type huntingtin; HD: Huntington's disease; STN: subthalamic nucleus

Wild-type HTT in the cortex appears critical for striatal health



Neuron Type	Genetic Status				Compartment
Cortical	WT	WT	HD	HD	Presynaptic
					Synaptic
Striatal	WT	HD	HD	WT	Post-synaptic
Network Status	*Functional*		*Dysfunctional*		

Status of the presynaptic compartment determines the integrity of the network

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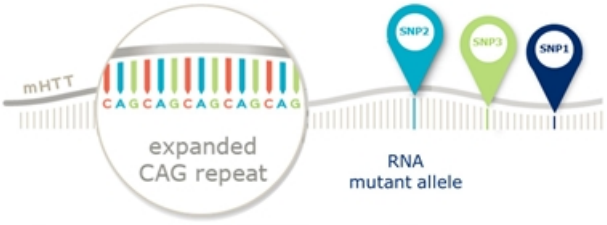
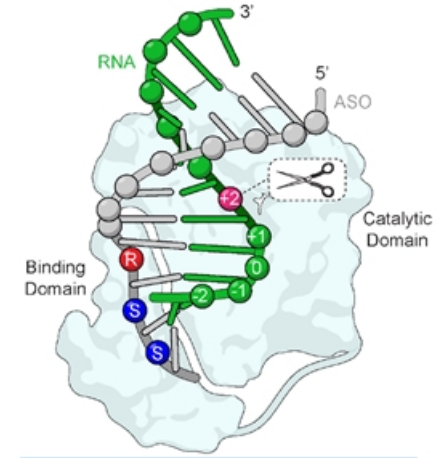
Presented by Dr. Frederic Saudou at Wave's Analyst and Investor Research Day on October 7, 2019
Virlogeux et al., Cell Reports 2018

Wave approach: novel, allele-selective silencing

Aims to lower mHTT transcript while leaving healthy wild-type HTT relatively intact

- Utilize association between single nucleotide polymorphisms (SNPs) and genetic mutations to specifically target errors in genetic disorders, including Huntington's disease (HD)
- Potential to provide treatment for up to 80% of HD population





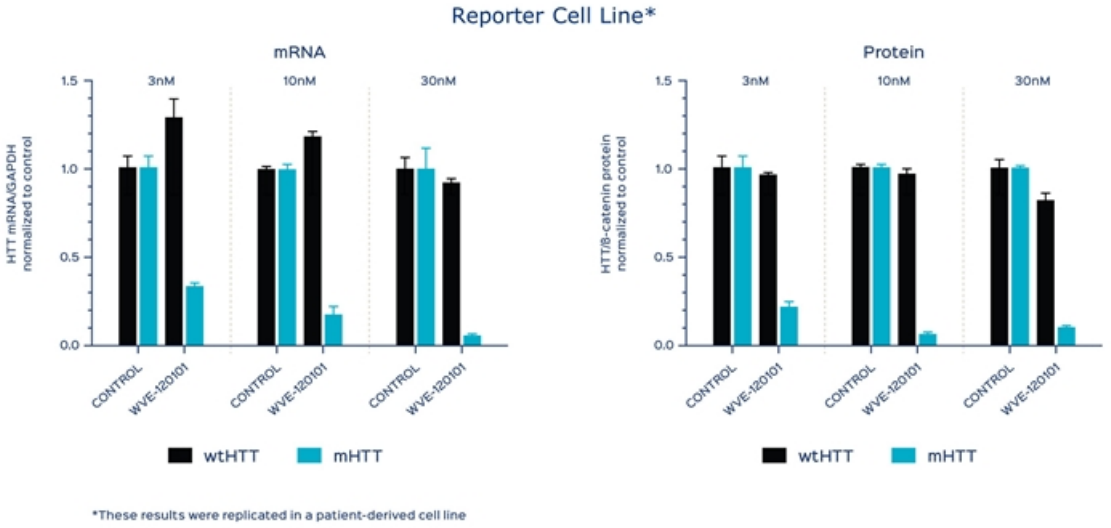
RNase H and ASO:RNA

Allele-selectivity possible by targeting SNPs associated with expanded long CAG repeat in HTT gene

WAVE LIFE SCIENCES

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

Selective reduction of mHTT mRNA & protein

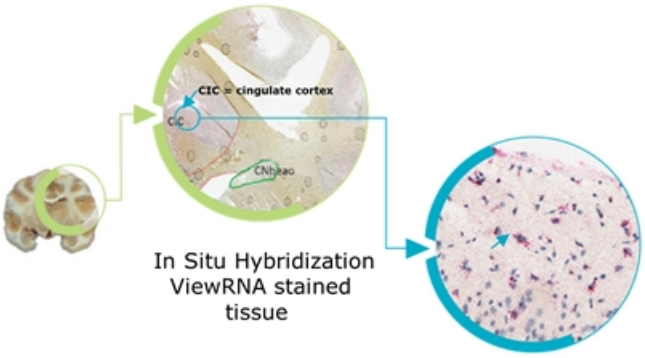


*These results were replicated in a patient-derived cell line

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

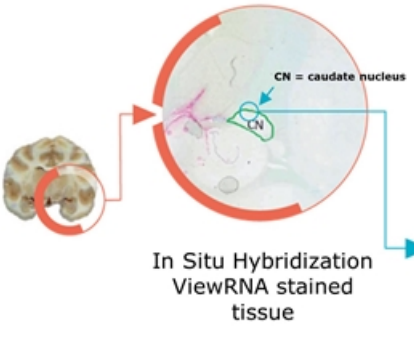
- WVE-120101 and WVE-120102 distribution in cynomolgus non-human primate brain following intrathecal bolus injection



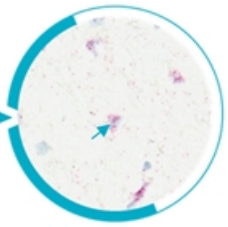
In Situ Hybridization ViewRNA stained tissue

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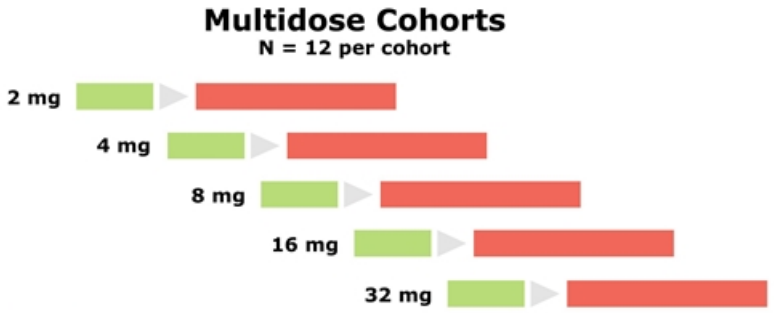
Source: Meena, Zboray L, Svrzikapa N, et al. Selectivity and biodistribution of WVE-120101, a potential antisense oligonucleotide therapy for the treatment of Huntington's disease. Paper presented at: 69th Annual Meeting of the American Academy of Neurology; April 28, 2017; Boston, MA.

PRECISION-HD clinical trial design

Two parallel, multicenter, double-blind, randomized, placebo-controlled Phase 1b/2a clinical trials for WVE-120101 and WVE-120102

PRECISION-HD2 OLE:
Initiated October 2019

PRECISION-HD1 OLE:
Initiated February 2020



- PRECISION-HD2 data from 32 mg cohort expected in 2H 2020
- PRECISION-HD1 topline data, including 32 mg cohort, expected in 2H 2020

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OLE: Open label extension; CSF: cerebrospinal fluid *Study day may vary depending on patient washout period

PRECISION-HD2 topline results

Clinical trial ongoing

Doses	Safety	Biomarker Effects	
		mHTT	wtHTT
• **WVE-120102 2–16 mg (pooled)**	• **Generally safe and well tolerated**	• **Reduction in mHTT compared to placebo (-12.4%[1], p<0.05[2])** • **Analysis across groups suggests dose response at highest doses (p=0.03)[3]**	• **No change in tHTT compared to placebo** • **Ongoing evaluation**
• 32 mg cohort initiated • Assessing the potential for higher dose cohorts	• Safety profile supports addition of higher dose cohorts	• Potential for greater mHTT reduction at higher doses	• Larger reductions of mHTT expected to result in discernible impact on tHTT

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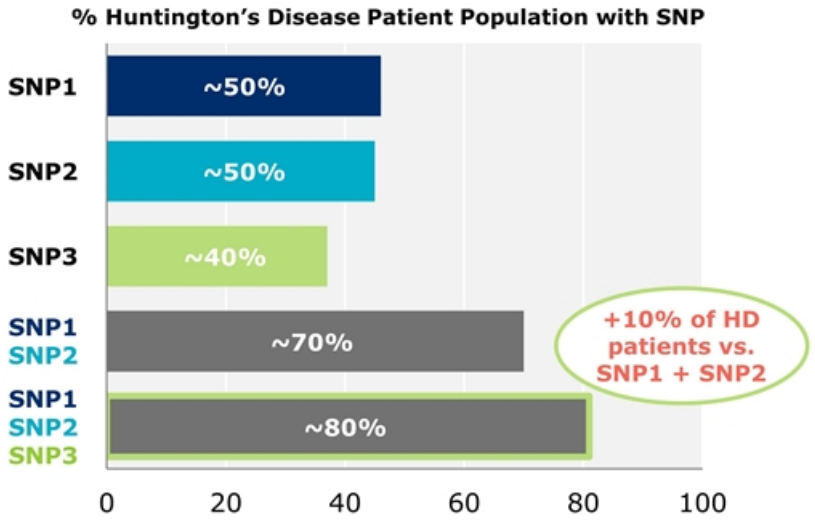
Topline results announced December 30. 2019; mHTT: mutant huntingtin wtHTT: wild-type HTT tHTT: total HTT
[1] Hodges-Lehmann non-parametric shift estimates of the difference between treatment and placebo; [2] Wilcoxon-Mann-Whitney non-parametric significance test; [3] Multiple Contrast Test (MCT)

Three allele-selective HD programs

Potential to address ~80% of HD patient population



Intend to explore efficacy in early manifest and pre-manifest HD patient populations

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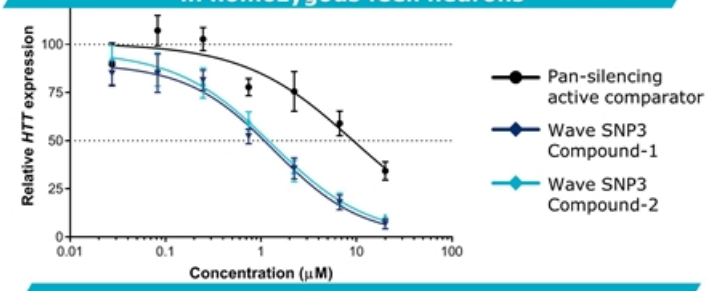
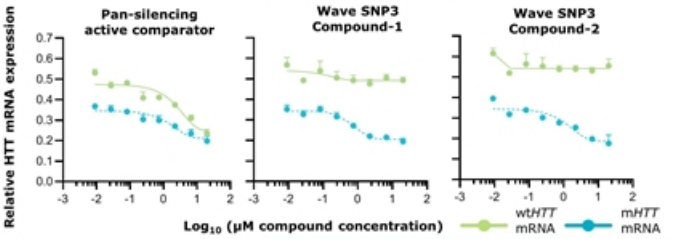
SNP3 program approaching clinical development

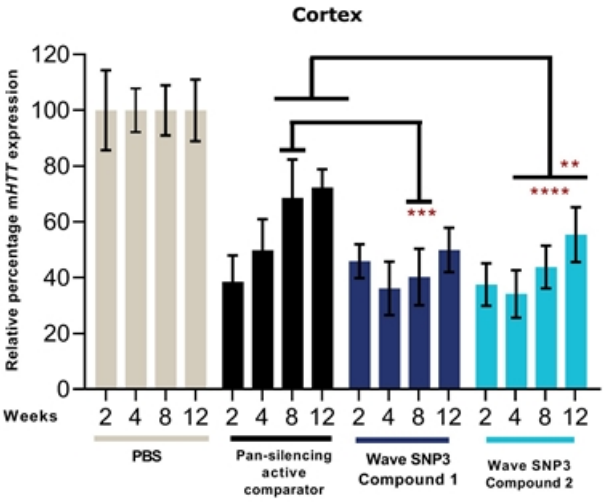
Potent mutant *HTT* knockdown activity in homozygous iCell neurons



No loss of selectivity with increasing concentrations



Knockdown persists for 12 weeks in BACHD mouse model



Similar knockdown achieved in striatum

Clinical development expected to initiate in 2H 2020

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Data presented at CHDI Foundation's 15th Annual HD Therapeutics Conference Feb 24-27, 2020; See poster for full dataset.
[Figure on right] Statistics: All oligo treatment groups statistically significantly different from PBS; One-way ANOVA ****, P≤0.0001. SNP3 Compound-1 and Compound-2 significantly different from pan-silencing active comparator at 8, 12 weeks ***, P<0.005; **P=0.001."

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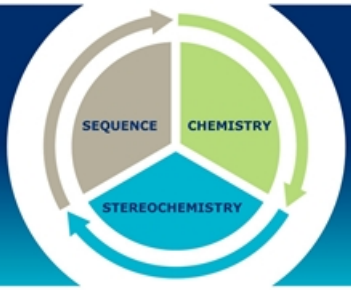
PRISM Platform

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

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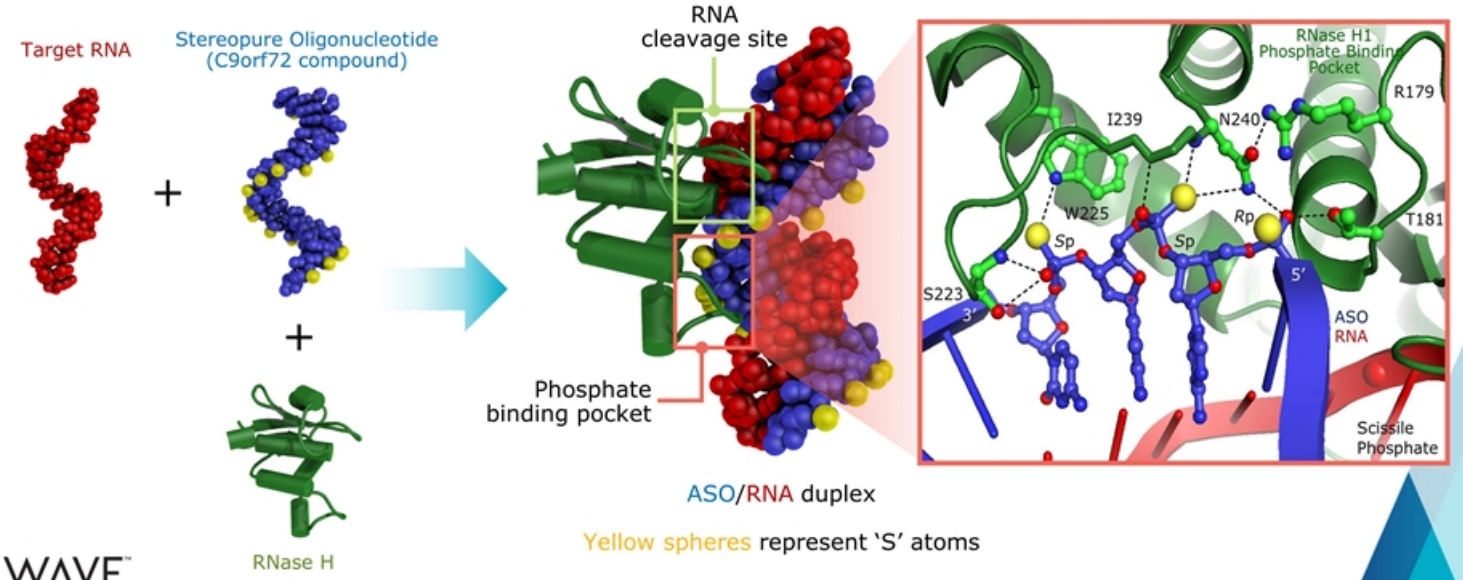
PRISM enables optimal placement of backbone stereochemistry

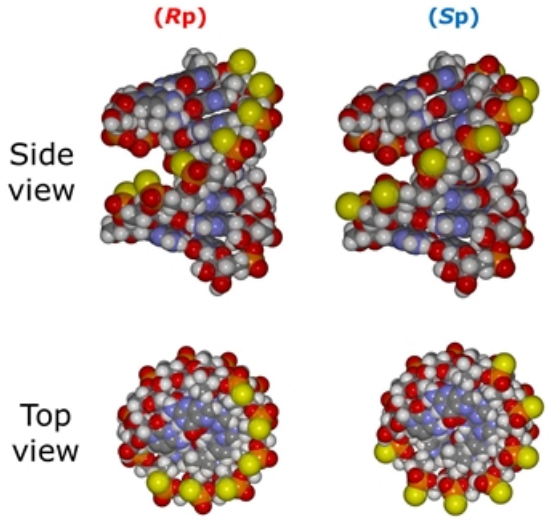
Crystal structure confirms phosphate-binding pocket of RNase H binds 3′-SSR-5′ motif in stereopure oligonucleotide – supports design strategy for Wave oligonucleotides



Yellow spheres represent 'S' atoms

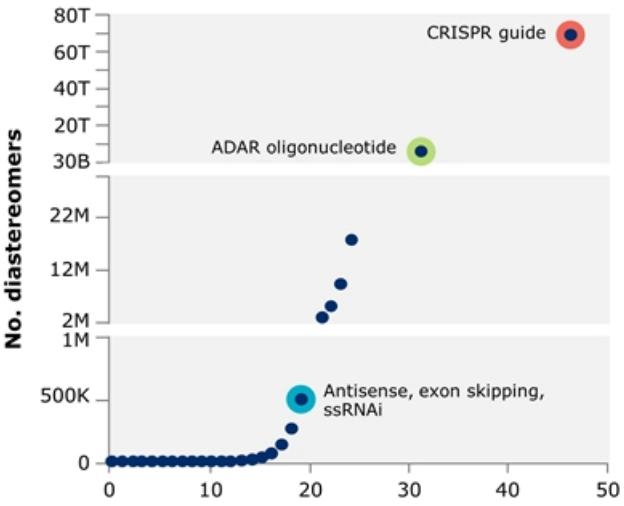
Importance of controlling stereochemistry

Stereochemical diversity



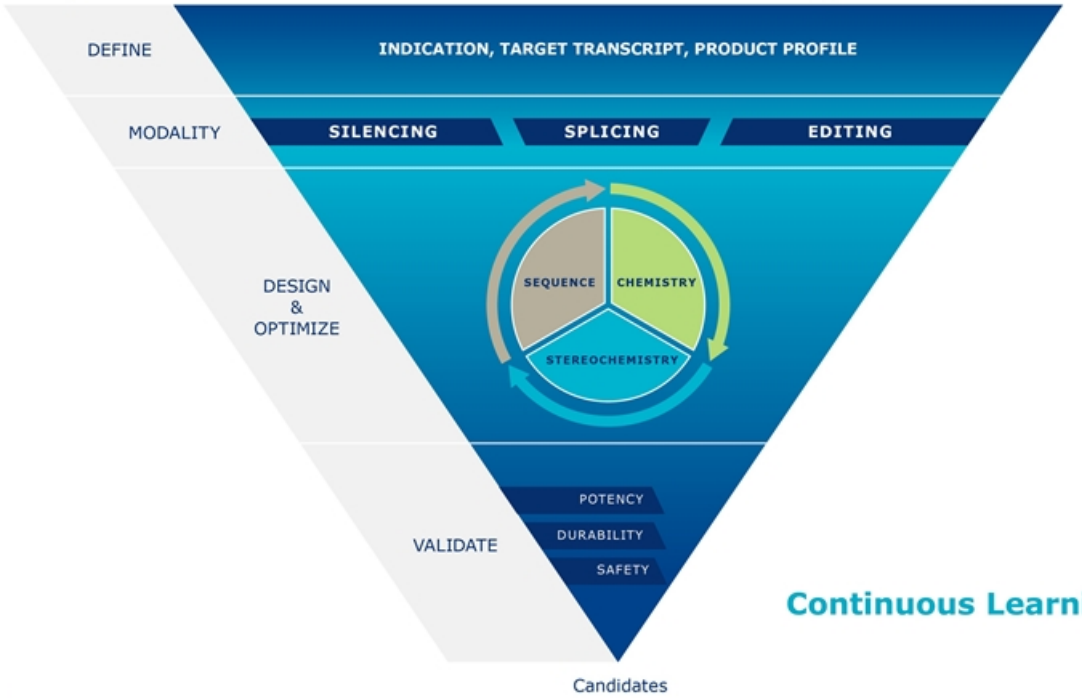
WAVE LIFE SCIENCES Yellow spheres represent 'S' atoms PS: Phosphorothioate

Exponential diversity arises from uncontrolled stereochemistry



Number of PS linkages in oligonucleotide backbone

PRISM platform enables rational drug design



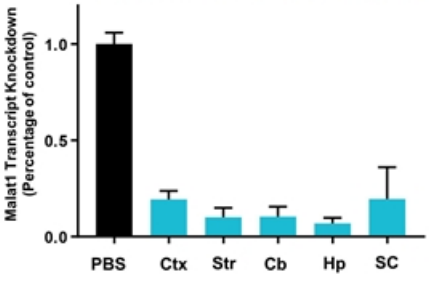
Continuous Learning

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

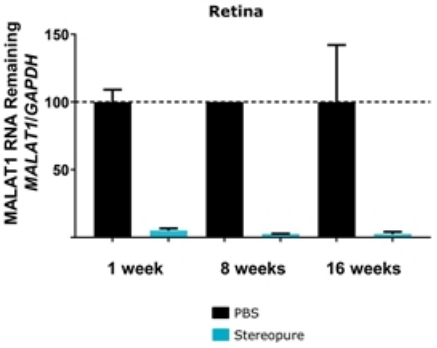
CNS

Malat1 Transcript Knockdown in Mice
10 Weeks after single 100 µg ICV injection



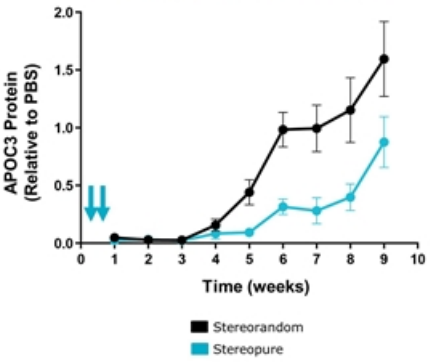
Eye

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



Liver

Knockdown of Serum APOC3 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.

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C9orf72 program

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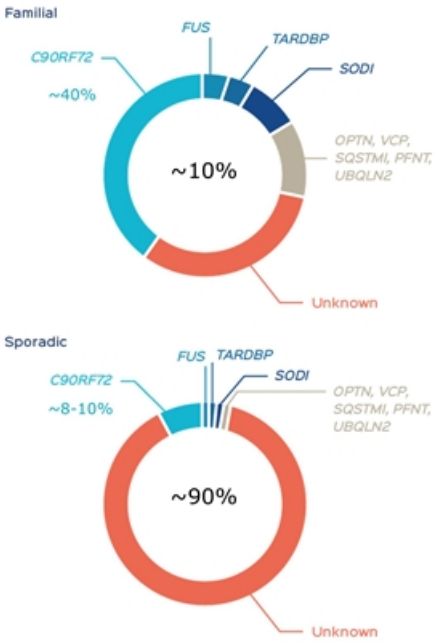
Source: DeJesus-Hernandez M, Mackenzie IR, Boeve BF, et al. *Neuron.* 2011;72:245-256. Renton AE, Majounie E, Waite A, et al. *Neuron.* 2011;72:257-268.

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



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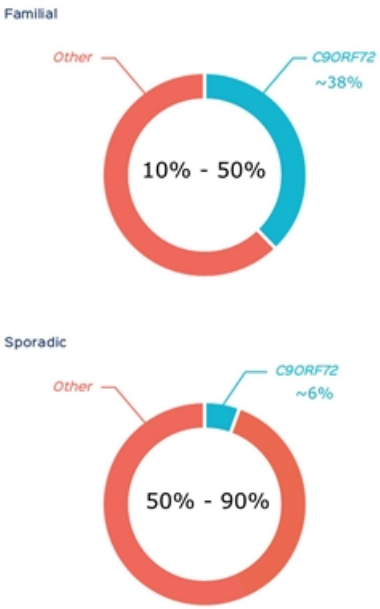
Source: Renton AE, Chiò A, Traynor BJ. State of play in amyotrophic lateral sclerosis genetics. *Nat Neurosci*. 2014;17:17–23.

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



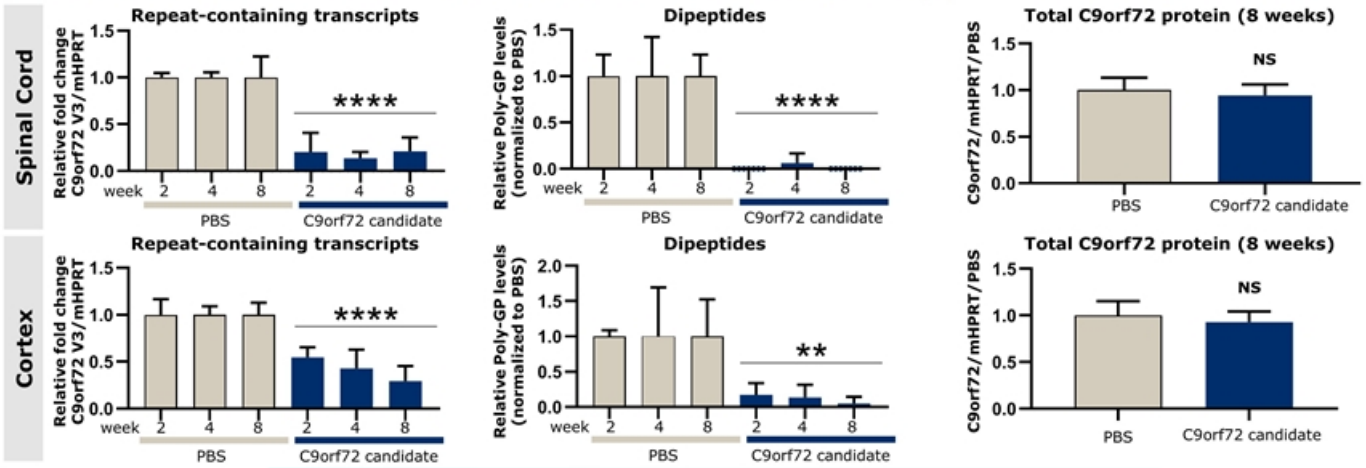
Sources: Stevens M, et al. Familial aggregation in frontotemporal dementia. *Neurology*. 1998;50:1541-1545. Majounie E, et al. Frequency of the C9orf72 hexanucleotide repeat expansion in patients with amyotrophic lateral sclerosis and frontotemporal dementia: a cross-sectional study. *Lancet Neurol.*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

C9orf72 program: Selective silencing *in vivo* of expanded C9orf72 repeat transcripts

- C9orf72 genetic mutations are the most common cause of familial Amyotrophic Lateral Sclerosis (ALS) and Frontotemporal Dementia (FTD) and are the strongest genetic risk factor found to date for the more common, non-inherited (sporadic) forms of ALS and FTD; Hexanucleotide repeat drives the formation and accumulation of dipeptide repeat proteins that accumulate in brain tissue
- **Wave's approach:** Selectively silence the repeat containing transcript while minimizing the impact on C9orf72 protein

Potent *in vivo* knockdown of repeat containing transcripts and dipeptides

Protein preservation



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Clinical development expected to initiate in 2H 2020

Experimental description: 2 x 50 ug on day 1 and day 8; mRNA Samples were analyzed using quantitative PCR (Taqman assay), Protein samples were measured by Western Blot. Dipeptide repeat proteins were measured by Poly-GP MSD assay.

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Ophthalmology

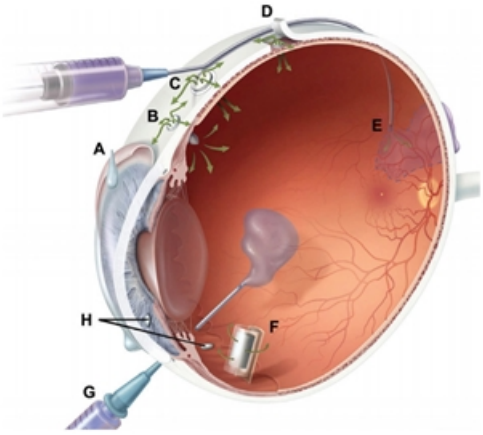
Stereopure oligonucleotides for inherited retinal diseases (IRDs)

Wave ophthalmology opportunity

- Oligonucleotides can be administered by intravitreal (IVT) injection; targeting twice per year dosing
- Stereopure oligonucleotides open novel strategies in both dominant and recessive IRDs; potential for potent and durable effect with low immune response

Successful targeting of *MALAT1* is a surrogate for an ASO mechanism of action

- Widely expressed in many different cell types
- Only expressed in the nucleus



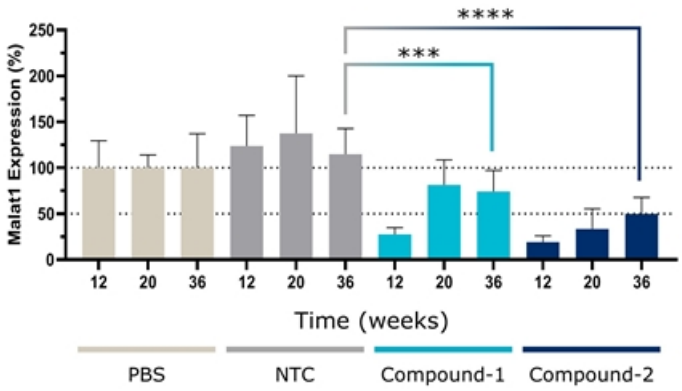
Intravitreal injection

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.; Short, B.G.; *Toxicology Pathology*, Jan 2008.

Stereopure compound induces potent and durable *MALAT1* knockdown in the eye

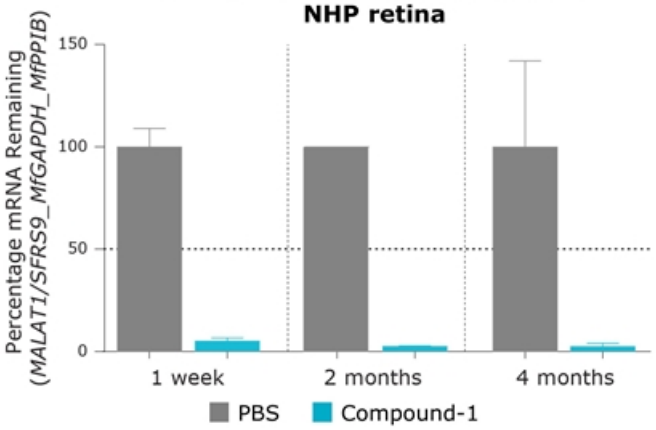
~50% *MALAT1* knockdown at 9 months

***In vivo* duration of effect in the mouse retina**



>90% knockdown of *MALAT1* maintained for 4 months

***In vivo* duration of effect in the NHP retina**



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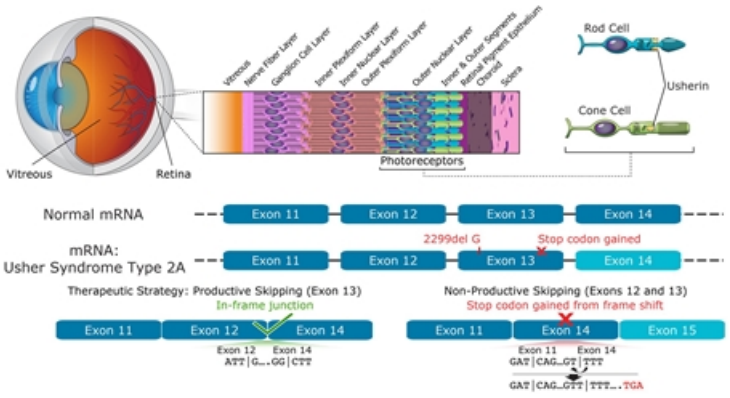
Mouse: Compound or PBS (1 x 50 mg IVT) was delivered to C57BL6 mice. Relative percentage of Malat1 RNA in the posterior of the eye (retina, choroid, sclera) to PBS-treated mice is shown at 12, 20 and 36 weeks post-single injection. Statistics: Compound-2 Malat1 levels are significantly different from NTC at 36 weeks ***, P<0.001; **** P<0.0001, respectively. PBS = phosphate buffered saline; NTC= chemistry matched non-targeting control; Compound-1 and Compound-2 are stereopure MALAT1-targeting antisense oligonucleotide. NHP: Oligonucleotide or PBS (1 x 450 µg IVT) was delivered to NHP. Relative percentage of *MALAT1* RNA in the retina to PBS-treated is shown at 1 week, 2 and 4 months, post-single injection. Compound-1 is a stereopure *MALAT1*-RNA-targeting antisense oligonucleotide.

Usher Syndrome Type 2A: a progressive vision loss disorder

- Autosomal recessive disease characterized by hearing loss at birth and progressive vision loss beginning in adolescence or adulthood
- Caused by mutations in USH2A gene (72 exons) that disrupt production of usherin protein in retina, leading to degeneration of the photoreceptors
- No approved disease-modifying therapies
- **~5,000 addressable patients in US**



Oligonucleotides that promote USH2A exon 13 skipping may restore production of functional usherin protein

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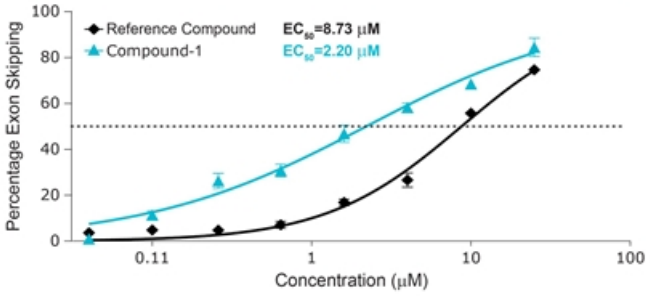
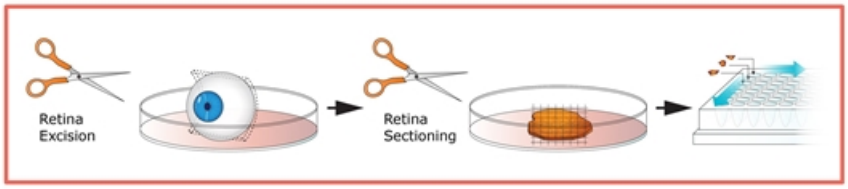
Sources: Boughman et al., 1983. *J Chron Dis.* 36:595-603; Seyedahmadi et al., 2004. *Exp Eye Res.* 79:167-173; Liu et al., 2007. *Proc Natl Acad Sci USA* 104:4413-4418.

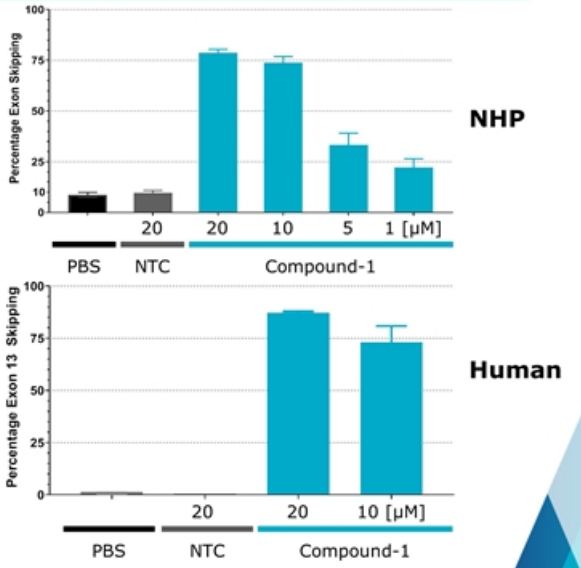
Potent USH2A exon 13 skipping with stereopure compound in *vitro* and *ex vivo*

Enhanced potency over a stereorandom reference compound (*in vitro*)





Target engagement in NHP and human retinas (*ex vivo*)



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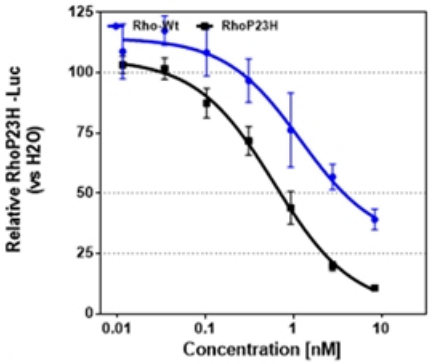
Left: Compounds were added to Y79 cells under free-uptake conditions. Exon skipping was evaluated by Taqman assays. *USH2A* transcripts were normalized to *SRSF9*. Data are mean±s.d., n=2. Reference Compound: van Diepen *et al.* 2018. Antisense oligonucleotides for the treatment of eye disease. W02018055134A1. Compound-1 is a stereopure antisense oligonucleotide. Right: Whole NHP and human eyes were enucleated (n=4 and n=2, respectively) and compounds (1–20 µM) were added to extracted retinas under free-uptake conditions. Exon skipping was evaluated by 48 hrs later by Taqman assays on RNA. USH2A transcript levels were normalized to SRSF9. Data presented are mean± s.e.m.

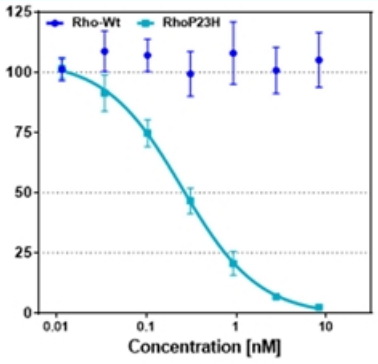
Allele-selective reduction of SNP-containing allele for adRP associated with Rhodopsin P23H mutation

- **Retinitis pigmentosa (RP)**: group of rare, genetic eye disorders resulting in progressive photoreceptor cell death and gradual functional loss; currently no cure
- ~10% of US autosomal dominant RP cases are caused by the P23H mutation in the rhodopsin gene (RHO)
- Mutant P23H rhodopsin protein is thought to misfold and co-aggregate with wild-type rhodopsin, resulting in a gain-of-function or dominant negative effect in rod photoreceptor cells

Stereorandom



Stereopure



In vivo

Collaborations in place for evaluation in transgenic human Rho P23H pig model

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Ferrari et al., *Current Genomics.* 2011;12:238-249.; Reporter assays on a Wave stereopure sequence as well as a sequence described in WO2016138353A1: ASO and luciferase reporter plasmids (wild-type and mutant rhodopsin) are transfected into Cos7 cells. 48-hours later, cells are harvested, and relative luminescence is measured.

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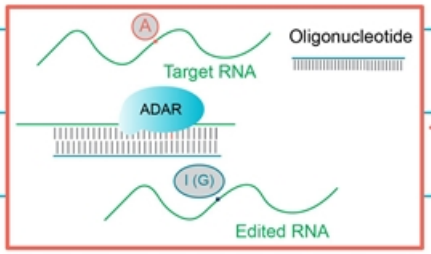
ADAR-mediated RNA editing

RNA-editing can be used for several therapeutic applications and supplement Wave's existing modalities

Strategy	Therapeutic Application	Treatment Modality: Silencing	Splicing	RNA Editing
Silence protein expression	Reduce levels of toxic mRNA/protein	✓		✓
Alter mRNA splicing	Exon skipping/inclusion/ restore frame		✓	✓
Fix nonsense mutations that cannot be splice-corrected	Restore protein expression			✓
Fix missense mutations that cannot be splice-corrected	Restore protein function			✓
Modify amino acid codons	Alter protein function			✓
Remove upstream ORF	Increase protein expression			✓



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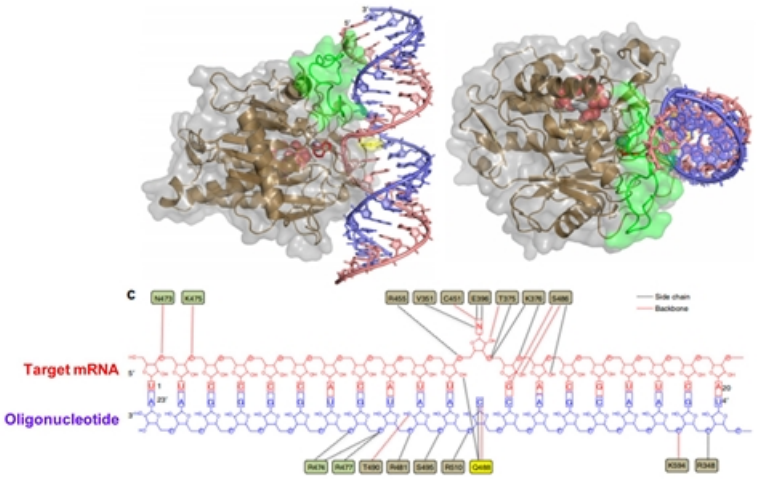
I (G): ADAR converts A>I, I is recognized as G by all cellular machinery; ADAR: Adenosine Deaminase Acting on RNA; ORF: Open reading frame

Using PRISM to unlock ADAR-mediated RNA editing

Structure of ADAR deaminase domain bound to dsRNA substrate



- ADAR makes multiple contacts with oligonucleotide backbone, sugar and bases
- Using PRISM platform, rationally designed and screened oligonucleotides to optimize:
  - 2' sugar chemistry
  - Backbone chemistry and stereochemistry
  - Size and structure
  - Modified nucleobases

~1,000 RNA editing oligonucleotides tested over the last year to develop SAR for editing format

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Structure adapted from Matthews et al., Nat Struct Mol Biol. (2016); SAR = structure-activity relationship; ADAR: Adenosine Deaminase Acting on RNA; dsRNA = double-stranded RNA

Wave's ADAR approach has several potential advantages over existing technologies

Existing RNA editing technologies		Wave's RNA editing platform
Use unmodified RNA	Stability	Fully chemically-modified stereopure oligonucleotides
Require AAV or lipid nano particle delivery	Delivery	Free uptake into tissues
Require exogenous protein (e.g. CAS13 or chimeric ADAR)	Editing	Uses endogenous ADAR for editing

Single oligonucleotide through free uptake is sufficient for editing

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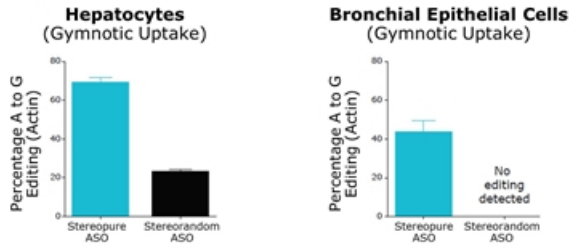
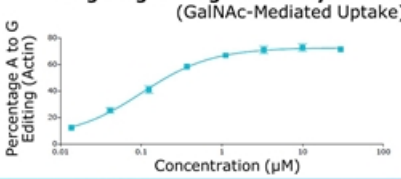
RNA Editing with Endogenous ADAR Achieved Across Multiple Primary Human Cell Types

Editing of Up to 70% Achieved *In Vitro*

Editing UAG Site in Actin mRNA in Primary Human Cell Lines



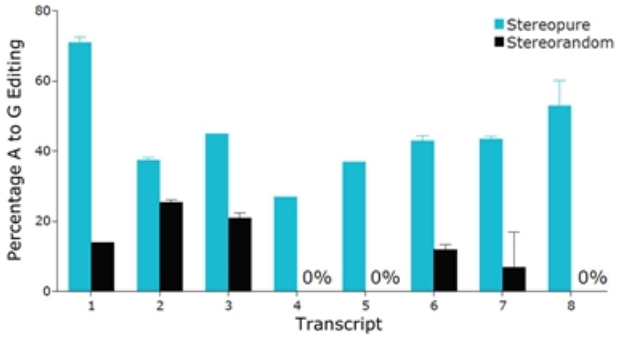
Targeting Editing in Primary Human Hepatocytes (GalNAc-Mediated Uptake)



- Dose dependent RNA editing with EC_{50} of ~100 nM

Technology Validated Across Multiple Sequences *In Vitro*

Editing in Primary Human Hepatocytes



- Editing achieved across several distinct RNA transcripts

In vivo editing data with fully modified stereopure oligonucleotides expected in 2020

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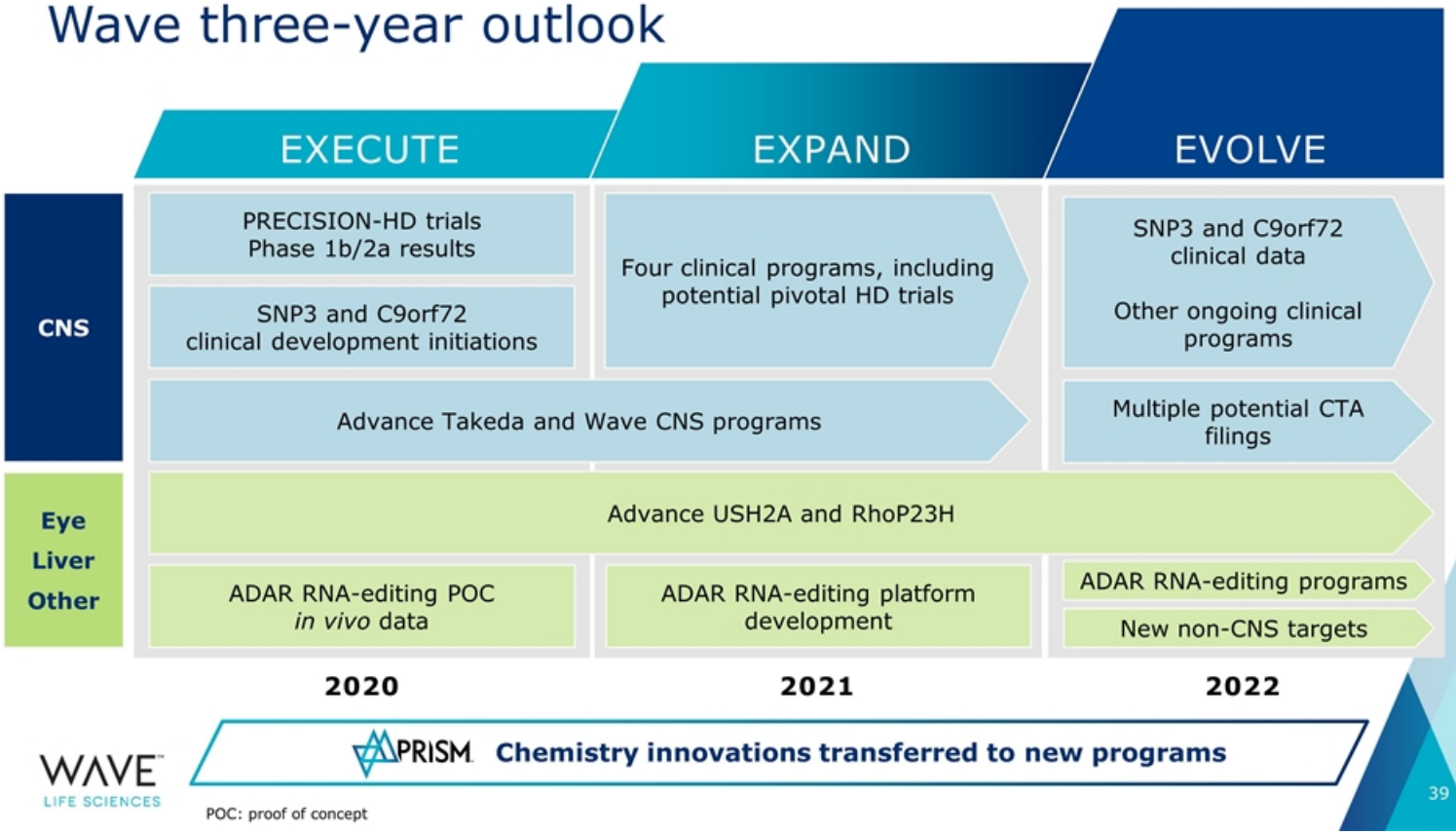
Data presented at 1st International Conference on Base Editing – Enzymes and Applications (Deaminet 2020); See poster for full dataset

Wave three-year outlook



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POC: proof of concept

Anticipated upcoming Wave milestones

CNS

- **2H 2020:** PRECISION-HD2 data from 32 mg cohort in Huntington's disease
- **2H 2020:** PRECISION-HD1 topline data, including 32 mg cohort, in Huntington's disease
- **2H 2020:** Initiate clinical development of SNP3 program in Huntington's disease
- **2H 2020:** Initiate clinical development of C9orf72 program in ALS and FTD

Ophthalmology

- **2020:** Advance USH2A and RhoP23H programs

RNA-editing

- **2020:** *In vivo* ADAR editing data

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Realizing the potential of genetic medicines

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