From Discovery to Deals: The Neurological Oligonucleotide Landscape at a Glance

Ioana Panait
RNA Research Lead
Ioana.panait@hansonwade.com

Matty Slingsby
Commercial Manager - RNA
Matty.slingsby@hansonwade.com

4th September 2025

Agenda

Intro to Beacon

Beacon adopts a modality approach to our database solutions, with custom built modules aligned to the core technologies across cutting edge research areas.
Each module is unique with customized search filters & searching ontologies, allowing you to ask deeper questions & gain more comprehensive insights on areas you are most invested in

Advanced Therapies

- Cell Therapy Beacon byhansonwade
- Gene Therapy Beacon byhansonwade
- RNA Beacon byhansonwade
- Cancer Vaccine Beacon byhansonwade
- Oncolytic Viruses Beacon byhansonwade

Beacon Biologics

- ADC Beacon byhansonwade
- Bispecific Beacon byhansonwade
- Checkpoint Beacon byhansonwade
- Cytokine Beacon byhansonwade

Beacon Small Molecules

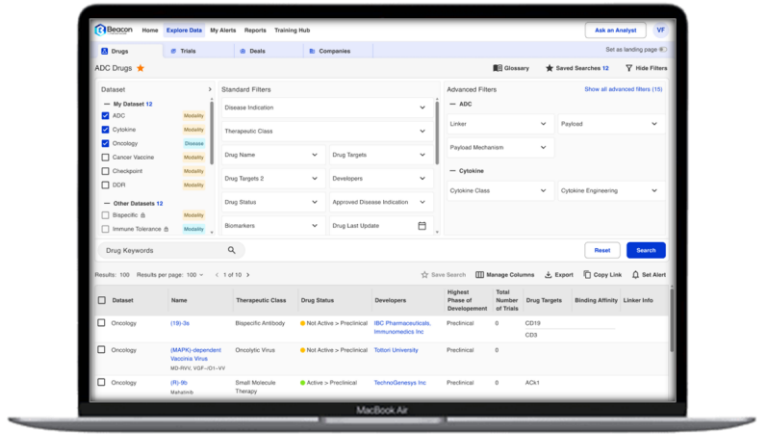
- Microbiome Beacon byhansonwade
- DDR Beacon byhansonwade
- TPD Beacon byhansonwade

Beacon Diseases

- Autoimmune Beacon byhansonwade
- Oncology Beacon byhansonwade

Newly Launched

- Neuroscience Beacon byhansonwade



Beacon RNA

BEACON RNA TRACKS OVER 5,600 DRUGS, 2,600 TRIALS, 4,000 DEALS & 3,300 COMPANIES

mRNA

mRNA Vaccines
mRNA-Based Antibody Production
mRNA-Based Protein Replacement
mRNA-Enhanced Cell Therapy
saRNA Therapies & Vaccines

OLIGONUCLEOTIDES

siRNA
miRNA
shRNA
Antisense Oligonucleotide (ASO)
Aptamers
Antibody Oligonucleotide Conjugates (AOC)

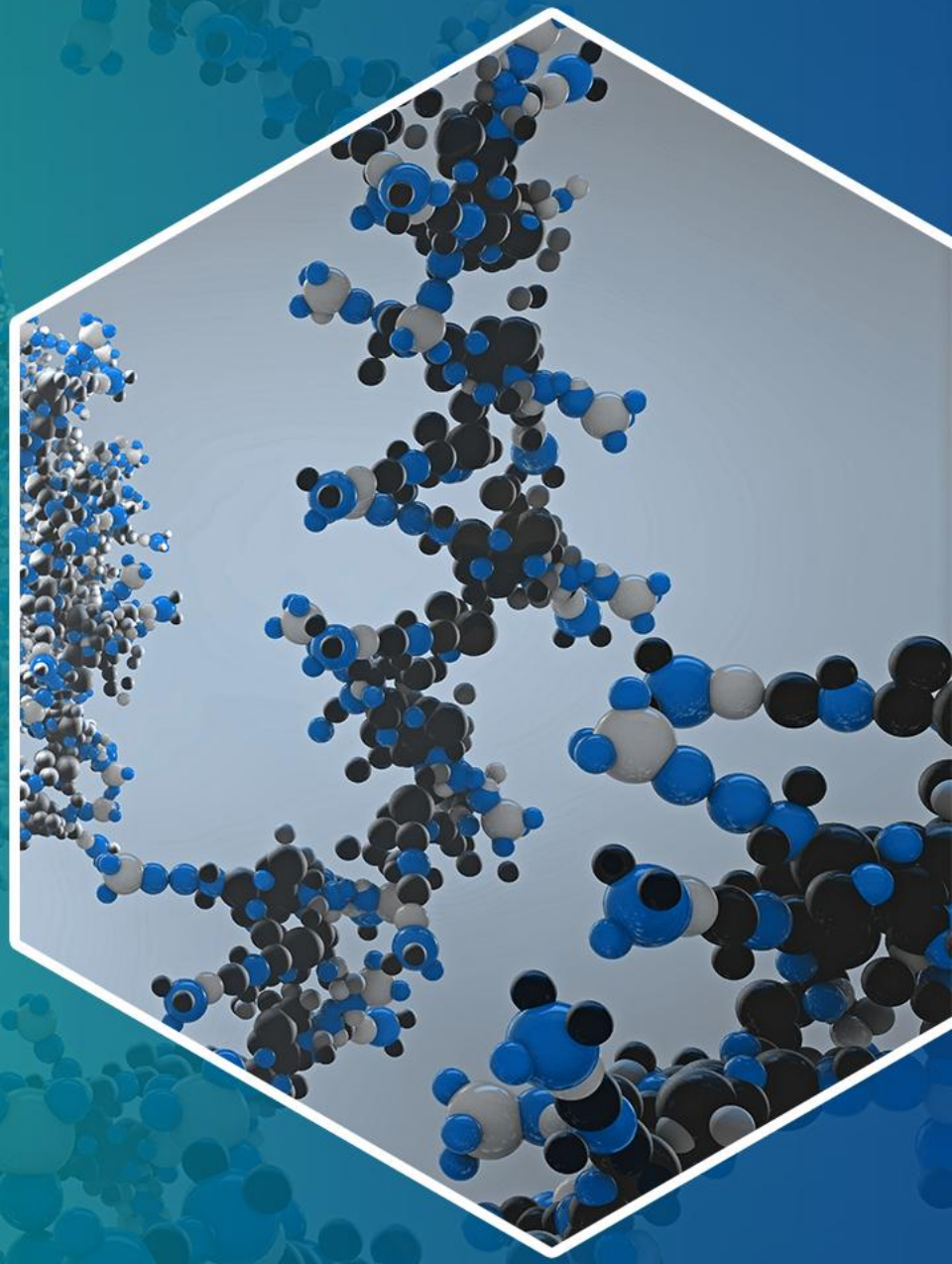
GENE EDITING

guide RNA mediated gene editing
CRISPR programs
Base Editing
RNA Editing
ADAR mediated editing

OTHER MODALITES

tRNA
Circular RNA
Ribozymes
RNA Targeting Small Molecules

Oligonucleotides for CNS – Why Now?



RNA Beacon byhansonwade

hansonwade

Recent Data in Neuroscience Field

66 New Drugs Added to Pipelines in 2025

- **28 ASO**
- **21 siRNA**

21 Clinical Trials Initiated in 2025

123 Active Clinical Trials

Stoke Therapeutics & Biogen Announced Data for ASO for Dravet Syndrome in Phase 1/2a trial

- A median reduction in seizures of 84.8% presented at IEC congress
- Phase 3 EMPEROR study now underway

Dyne Therapeutics announced Clinical Data for AOC targeting DMD at various conferences

- DYNE-251 led to improvements across multiple functional endpoints
- DYNE-251 drove dose-dependent increases in mean PMO muscle concentration and exon skipping

Alnylam Released Preclinical Data for Mivelsiran, 16C conjugated siRNA for Alzheimer's Disease

- Mouse model data at AAIC showed APP-lowering siRNA reduced disease pathology in 5xFAD mode
- Dose dependant lowering of amyloid in tissue, glial inflammation & plasma NfL to support clinical development

Voyager Therapeutics Presented Preclinical Updates for Their siRNA AAV therapy for Alzheimer's

- Tau mRNA levels were reduced by greater than 50%, and total tau protein levels were reduced by 40-55% at 11 weeks post-injection
- TRACER platform supports CNS specific targeting. Voyager also working on non viral BBB shuttle NeuroShuttle ™

Recent Oligonucleotide Deals in CNS

Arrowhead & Novartis Enter Licensing Agreement for siRNA Therapy for Parkinson's + Additional Targets

- $200m upfront payment, up to $2bn in milestone payments
- Use of Arrowhead's proprietary TRiM™ platform for subcutaneous CNS delivery

Leal Therapeutics Announced $30m Series A for Neurodegenerative & Neuropsychiatric Disorders

- Pipeline spans small molecule for psychiatric disorders & ASO for ALS
- Leal also advancing next generation technology to optimize nucleic acid delivery to CNS through BBB shuttles

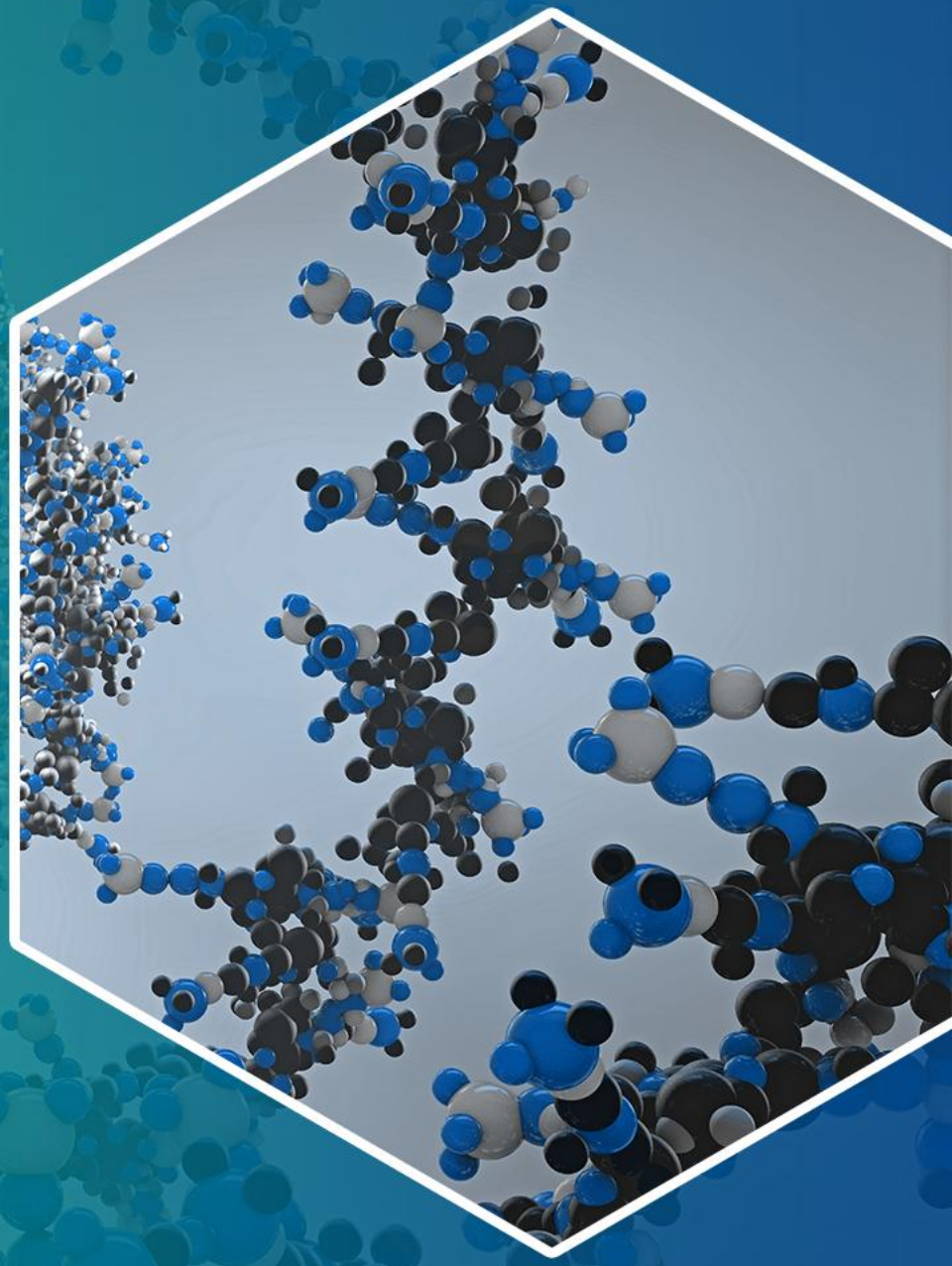
Secarna & Vect-Horus Partner to Advance Systemic Delivery of Oligonucleotides for CNS

- Combining Vect-Horus' VECTrans® delivery platform with Secarna's OligoCreator® technology
- VHH Antibody delivery platform to shuttle oligo payloads across BBB

Biogen & City Therapeutics Announce RNAi Collaboration for CNS Diseases

- Utilises City Therapeutics' RNAi expertise & ligand platform to target CNS
- Initial program used tissue enhanced delivery for systemic administration

The Oligos for CNS Landscape – Preclinical Clinical and Regulatory Insights



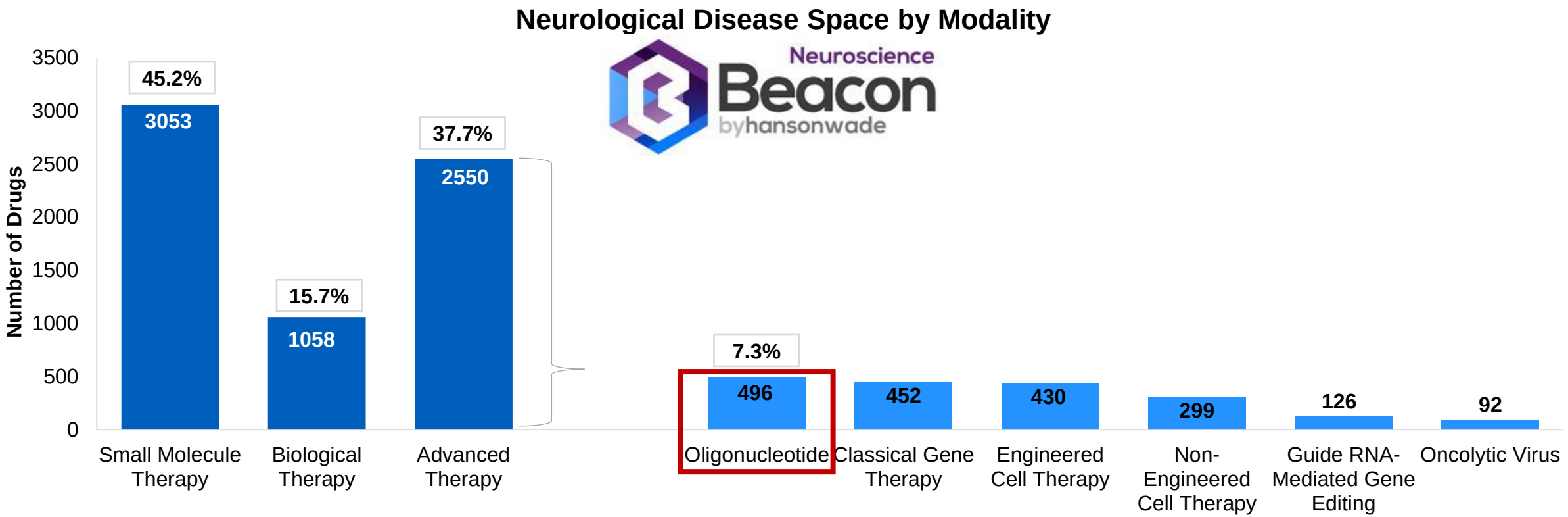
RNA Beacon byhansonwade

hansonwade

Overview: The Neurology Space

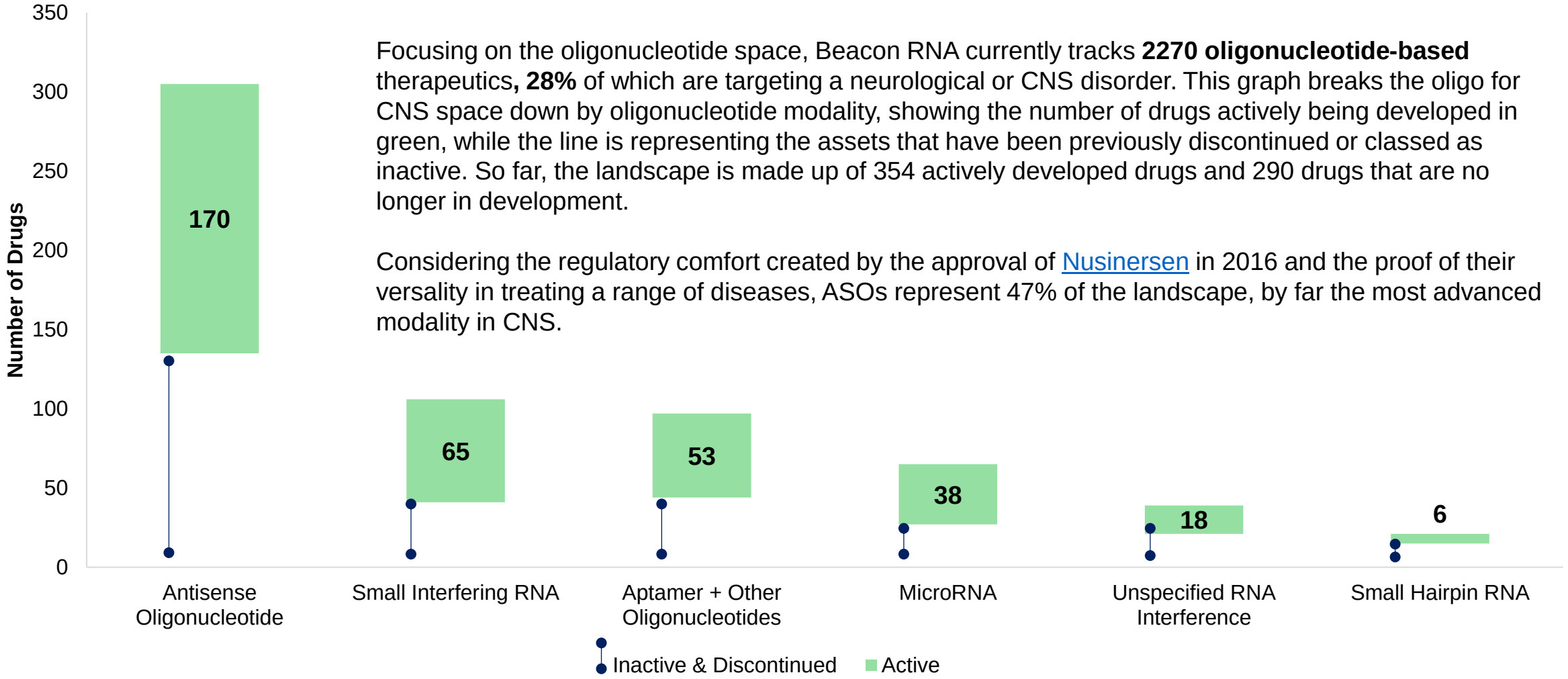
Looking at the overall neurology space, it's noticeable that 6000+ drugs are currently targeting neurological disorders. Of these, almost 500 are oligonucleotide-based drugs, representing 7% of the neurological disorder landscape. This data has been extracted from our newest Beacon Dataset, Beacon Neuroscience, which will launch on September 2nd. For more details, get in touch **here.**

Oligonucleotide-based drugs are the most frequently leveraged of all advanced therapies for treating CNS disorders as they can precisely modulate genetic drivers, have durable effects and carry regulatory precedent.



Oligonucleotide Modality Distribution

Oligonucleotides for CNS by Modality

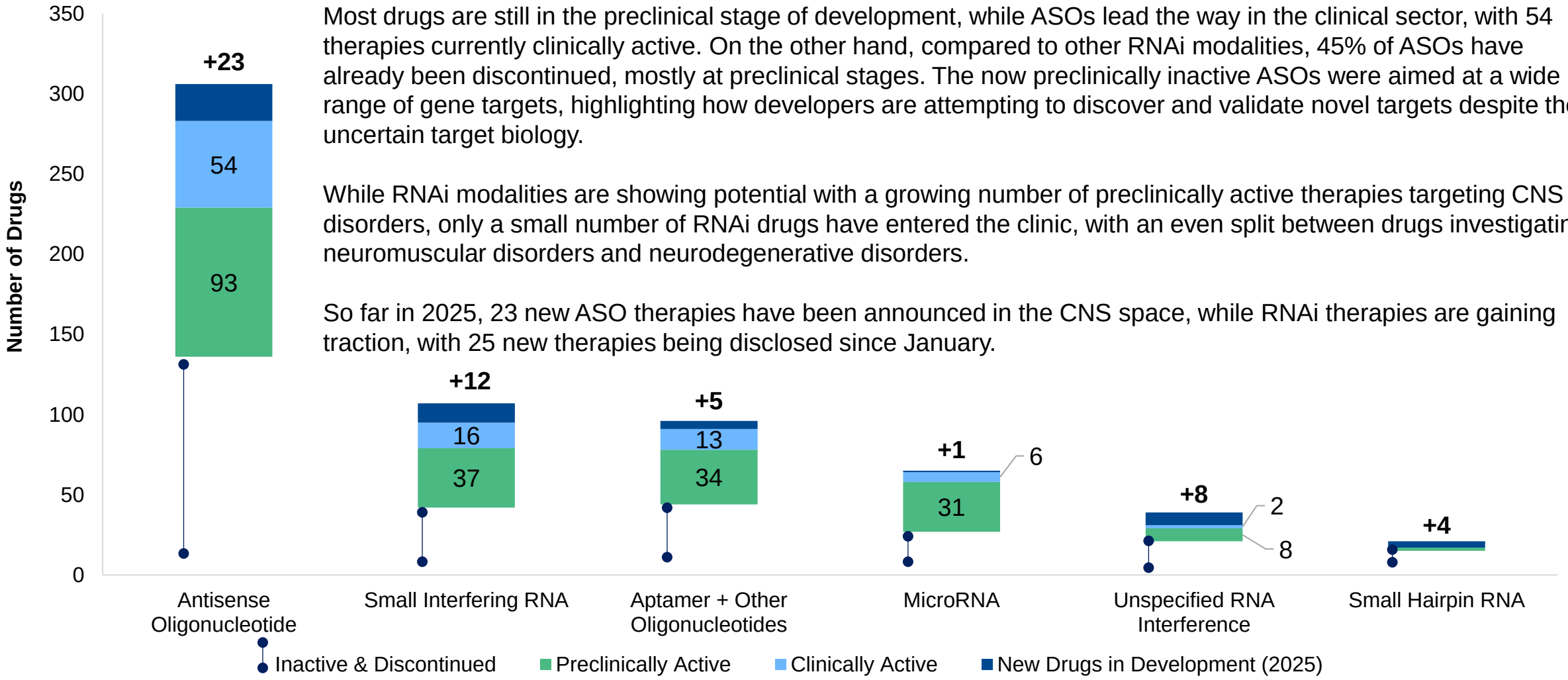


Focusing on the oligonucleotide space, Beacon RNA currently tracks **2270 oligonucleotide-based** therapeutics**, 28%** of which are targeting a neurological or CNS disorder. This graph breaks the oligo for CNS space down by oligonucleotide modality, showing the number of drugs actively being developed in green, while the line is representing the assets that have been previously discontinued or classed as inactive. So far, the landscape is made up of 354 actively developed drugs and 290 drugs that are no longer in development.

Considering the regulatory comfort created by the approval of Nusinersen in 2016 and the proof of their versality in treating a range of diseases, ASOs represent 47% of the landscape, by far the most advanced modality in CNS.

Oligonucleotide Status Distribution

Oligonucleotides for CNS by Modality and Status

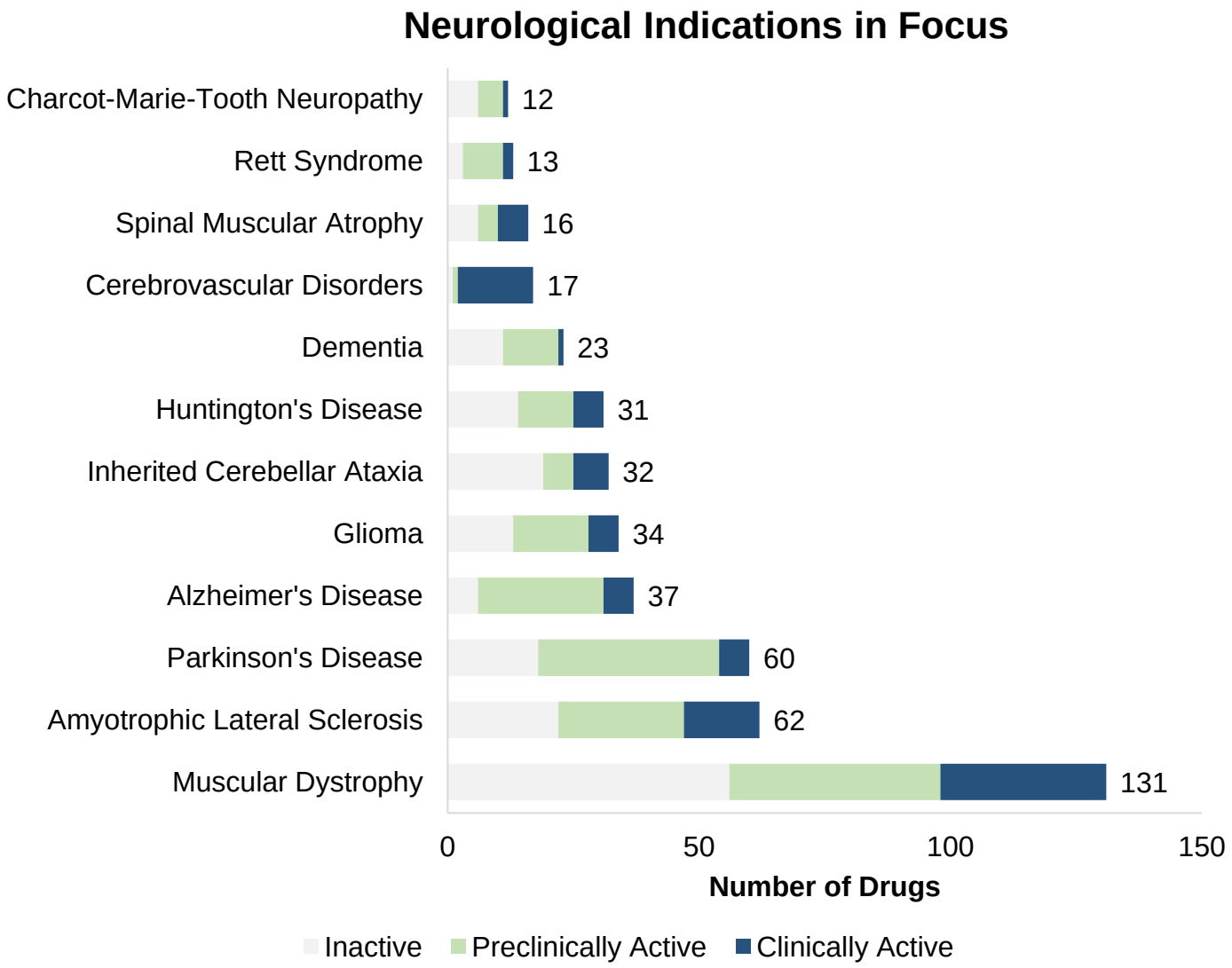


Most drugs are still in the preclinical stage of development, while ASOs lead the way in the clinical sector, with 54 therapies currently clinically active. On the other hand, compared to other RNAi modalities, 45% of ASOs have already been discontinued, mostly at preclinical stages. The now preclinically inactive ASOs were aimed at a wide range of gene targets, highlighting how developers are attempting to discover and validate novel targets despite the uncertain target biology.

While RNAi modalities are showing potential with a growing number of preclinically active therapies targeting CNS disorders, only a small number of RNAi drugs have entered the clinic, with an even split between drugs investigating neuromuscular disorders and neurodegenerative disorders.

So far in 2025, 23 new ASO therapies have been announced in the CNS space, while RNAi therapies are gaining traction, with 25 new therapies being disclosed since January.

Neurological Indications in Focus



This graph plots the most frequently targeted neurological indications, segmented by drug status. As expected, this shows muscular dystrophies to be clinically and preclinically the most investigated diseases in the space. Compared to neurodegenerative disorders, muscular disorders are caused by monogenic targets and don't face the challenge of having to cross the blood-brain barrier for effective treatment, resulting in muscular dystrophies being well-represented in ongoing clinical programs.

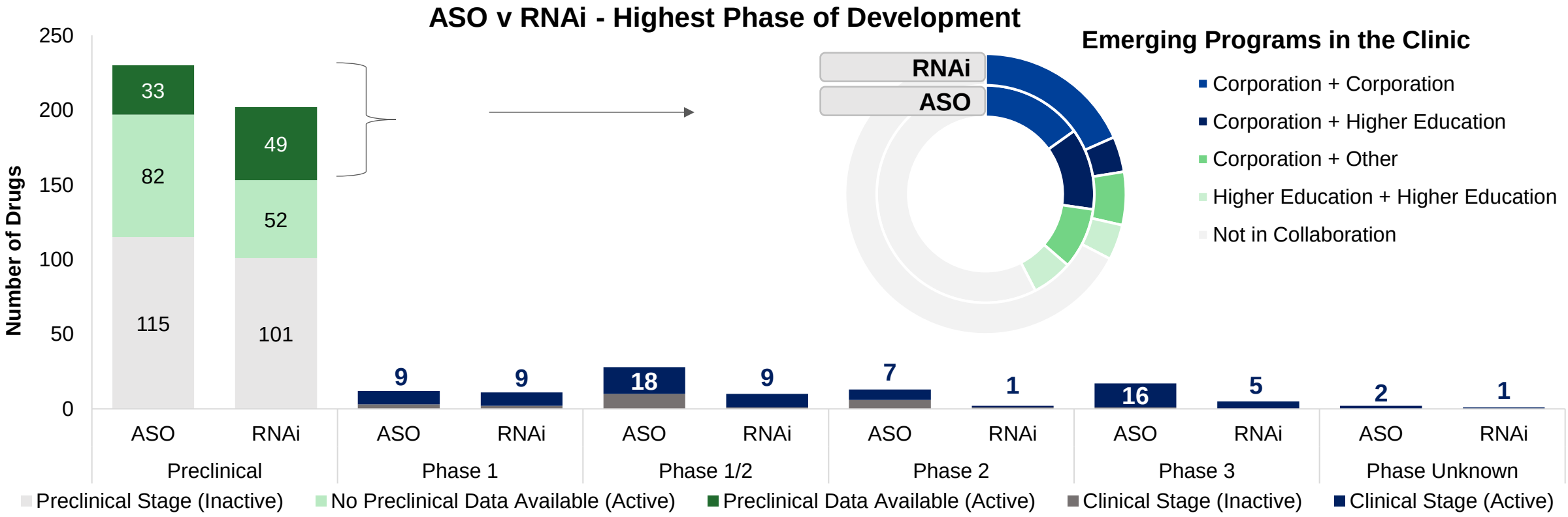
Looking beyond muscular dystrophies, Amyotrophic Lateral Sclerosis (ALS), Parkinson's, and Alzheimer's disease (AD) have significant preclinical maturity. ALS is the most extensively studied neurodegenerative disorder in clinical stages, as 15 drugs are actively investigated in the clinic. The most advanced of these programs is ulefnersen, currently investigated in phase 3 trials.

Huntington's disease (HD) and ALS programs have high rates of interrupted development, particularly in early clinical testing. This snapshot reflects the target validation risk, and the fact that programs targeting complex diseases are currently more likely to fail.

ASO vs RNAi: Phase of Development

This graph highlights the availability of preclinical data for the two modalities in the context of the current landscape based on the phases of development reached. The data shows that a similar number of ASO vs RNAi programs have been dropped at preclinical stages, while a higher proportion of preclinically active RNAi programs have generated publicly released data.

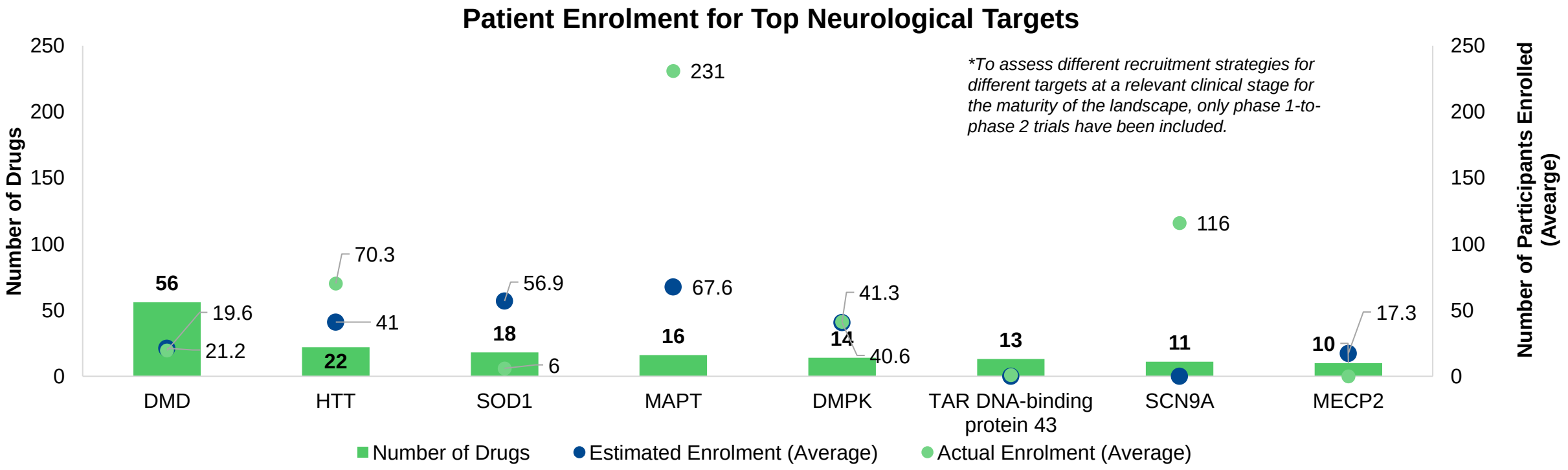
Moreover, this data helps us determine potentially emerging programs. The pie chart shows that, of those with preclinical data generated, 5 ASO programs and 9 RNAi programs, are being developed in collaboration by 2+ corporations. This is a promising sign that these programs may have enough funding to advance into the clinic.



Top Neurological Targets

This graph shows the most popular neurological drug targets in the oligonucleotide landscape, plotted against the average estimated and current enrolment figures pulled from the trials associated with these respective genes.

While DMD is the most investigated drug target both preclinically and in the clinic, the patient pool is smaller as opposed to other diseases such as HD and ALS. However, when it comes to HD, even though only seven trials were eligible for this analysis, clinical studies typically target cognitive and functional endpoints due to the progressive nature of the disease, requiring a larger sample size to achieve statistical significance. Finally, for SOD1, the main ALS target, average current enrolment appears to be underestimated by 50 people. This is influenced by the fact that one ALS trial has been withdrawn early before enrolling patients, a phase 1 trial from Voyager Therapeutics investigating VY-HTT01.

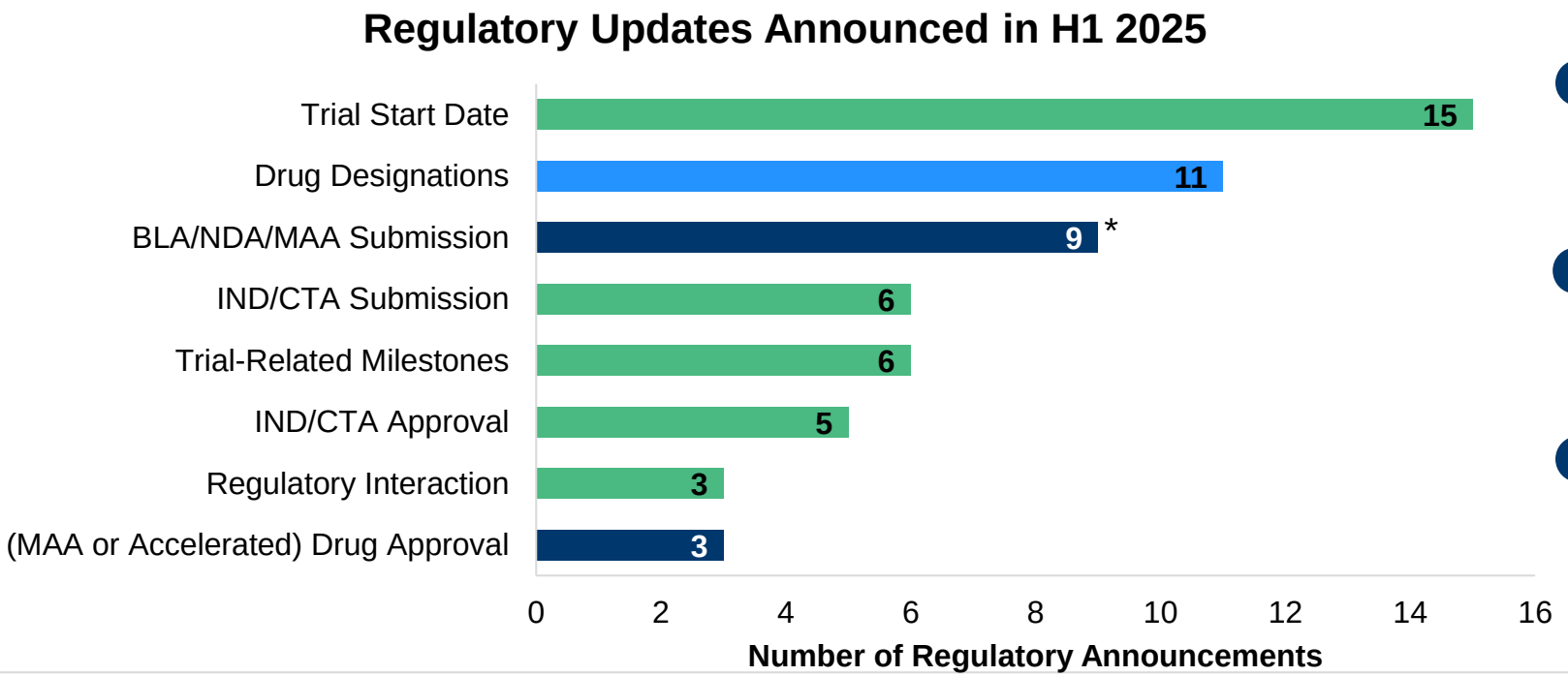


Regulatory Milestones in H1 2025

Regulatory announcements disclosed in H1 2025 have been listed based on the type of update.

H1 2025 reflects the current state of the sector, with a large proportion of regulatory milestones seen signalling a push towards advancing further into clinical research. Trial initiation remains top of the list, followed by granted drug designations playing an impact into the acceleration of clinical development that comes with financial incentives and frequent review of data by FDA.

A few key regulatory milestones from this year have been listed here.



Dyne Therapeutics
Breakthrough Therapy and Fast Track Designation to DYNE-101 for the treatment of MDT1

Biogen
US FDA has granted Fast Track designation to BIIB080 for the treatment of Alzheimer's disease

Avidity Biosciences
Plans marketing application submissions for delpacibart etedesiran in 2026 (US & EU) for DM1

uniQure
Plans to submit a BLA for AMT-130 for the treatment of Huntington's disease in Q1 2026

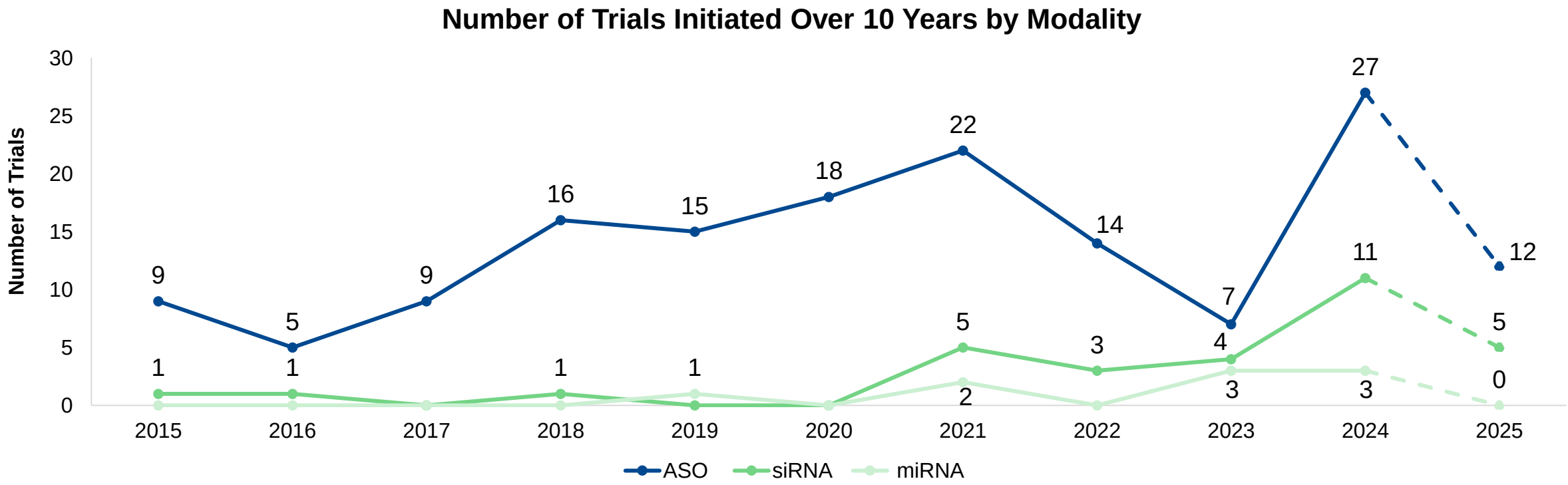
Wave Life Sciences
Plans to file an NDA for the accelerated approval of WVE-N531 for DMD in 2026

*This includes expected milestone with a future date.

Current Trial Landscape

Beacon RNA tracks 242 trials in the oligonucleotide space for CNS disorders. Here, the trial landscape is broken down by modality. The clinical dominance of ASOs has been well established over the past 10 years. The first half of 2025 saw the initiation of 12 ASO trials and five siRNA trials. ASO momentum continues; earlier this year, the clinical hold was lifted for Amylyx's AMX0114. At the same time, priority regulatory designations are expected to lead to faster development timelines, and the maturity of this modality from experimental to mainstream clinical development, which may also eventually lead to a value surge.

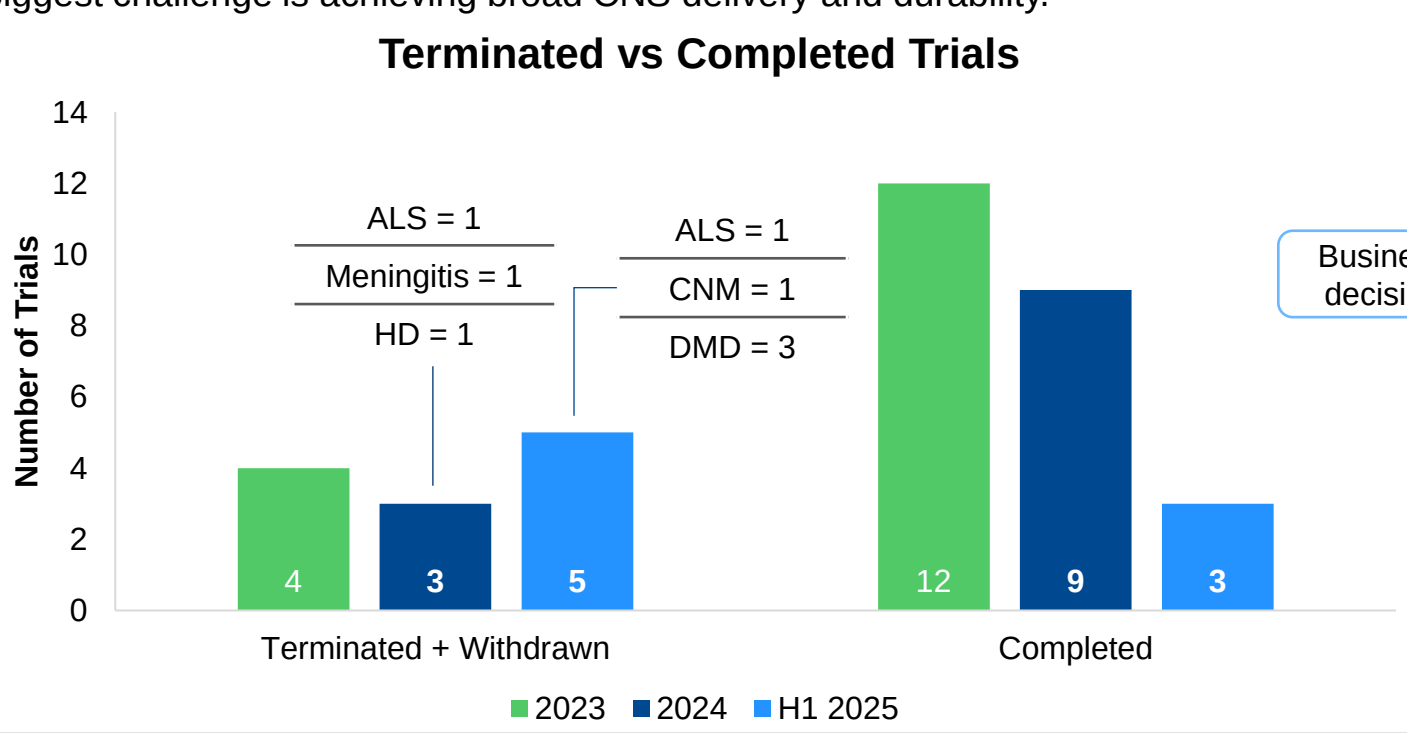
While siRNAs remain largely preclinical, reflecting delivery challenges and fewer clinical entrants, since 2020 there has been a rise in clinical trials initiated investigating RNAi assets.

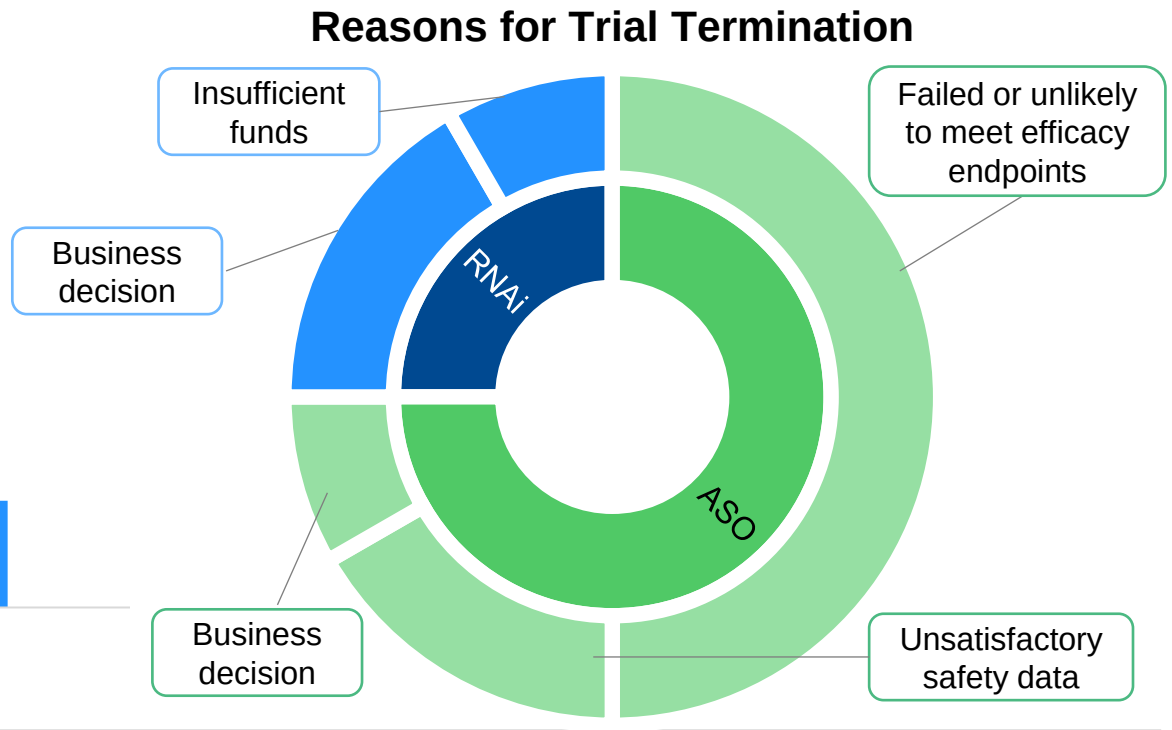


Terminated vs Completed Trials

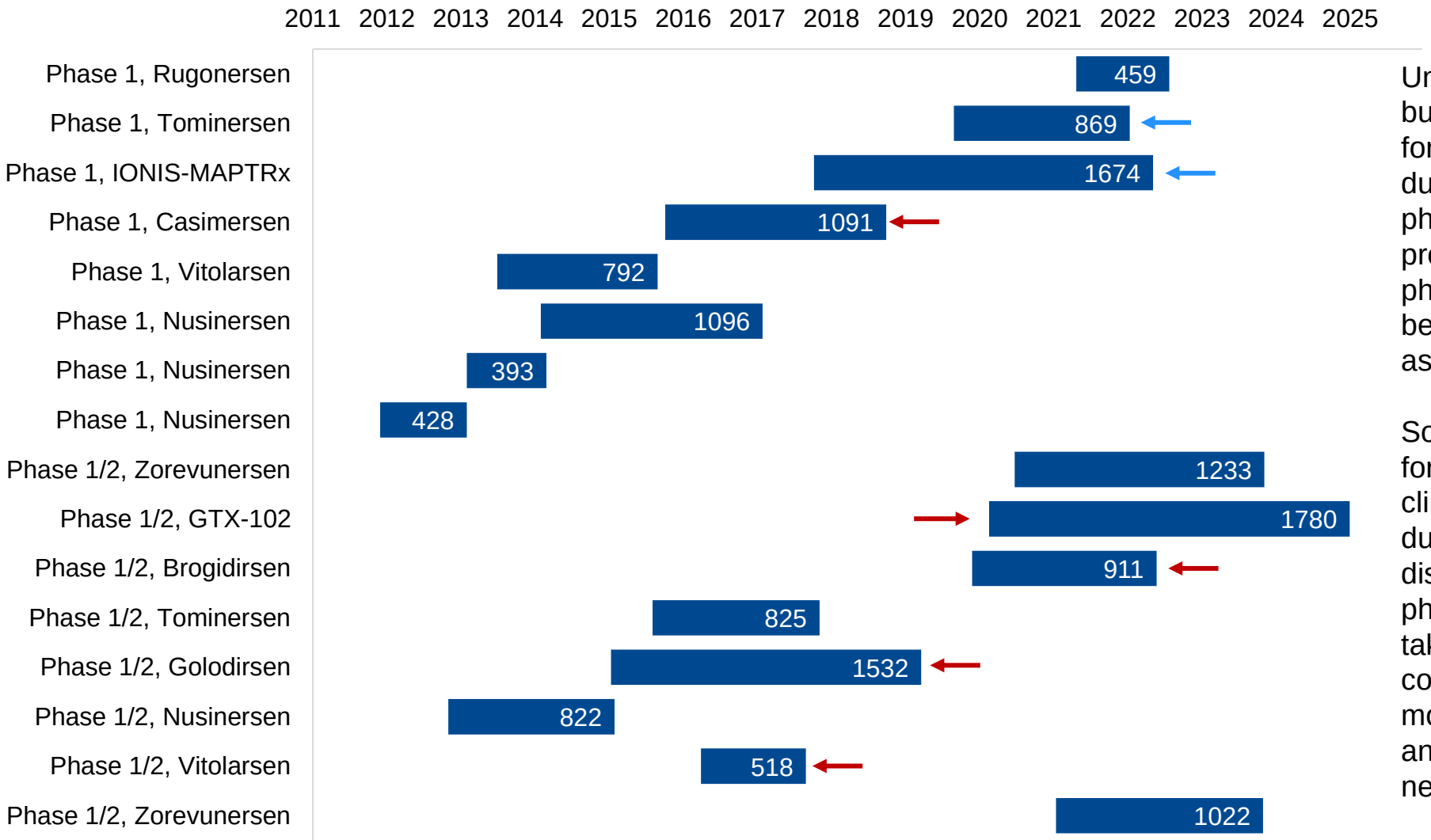
The number of terminated or withdrawn vs. completed trials in the space over the past two years is displayed here. In the oligo for CNS space, the number of trials that have been terminated has remained steady, however there were five trials terminated only in H1 2025. In comparison, a significantly larger volume of trials get marked as completed, highlighting the positive trajectory of this space, with 16 more trials expected to be completed by the end of the year.

Moreover, the pie chart highlights the reason for early trial termination from 2023 onwards. These vary depending on modality; for ASOs in clinical stages, failures are often tied to a lack of efficacy in complex neurodegenerative diseases (e.g., HD, ALS). siRNAs have seen fewer terminations simply because fewer trials exist. While the disclosed reasons for termination are currently related to business decisions, it is well known that the biggest challenge is achieving broad CNS delivery and durability.





Successfully Completed Trials

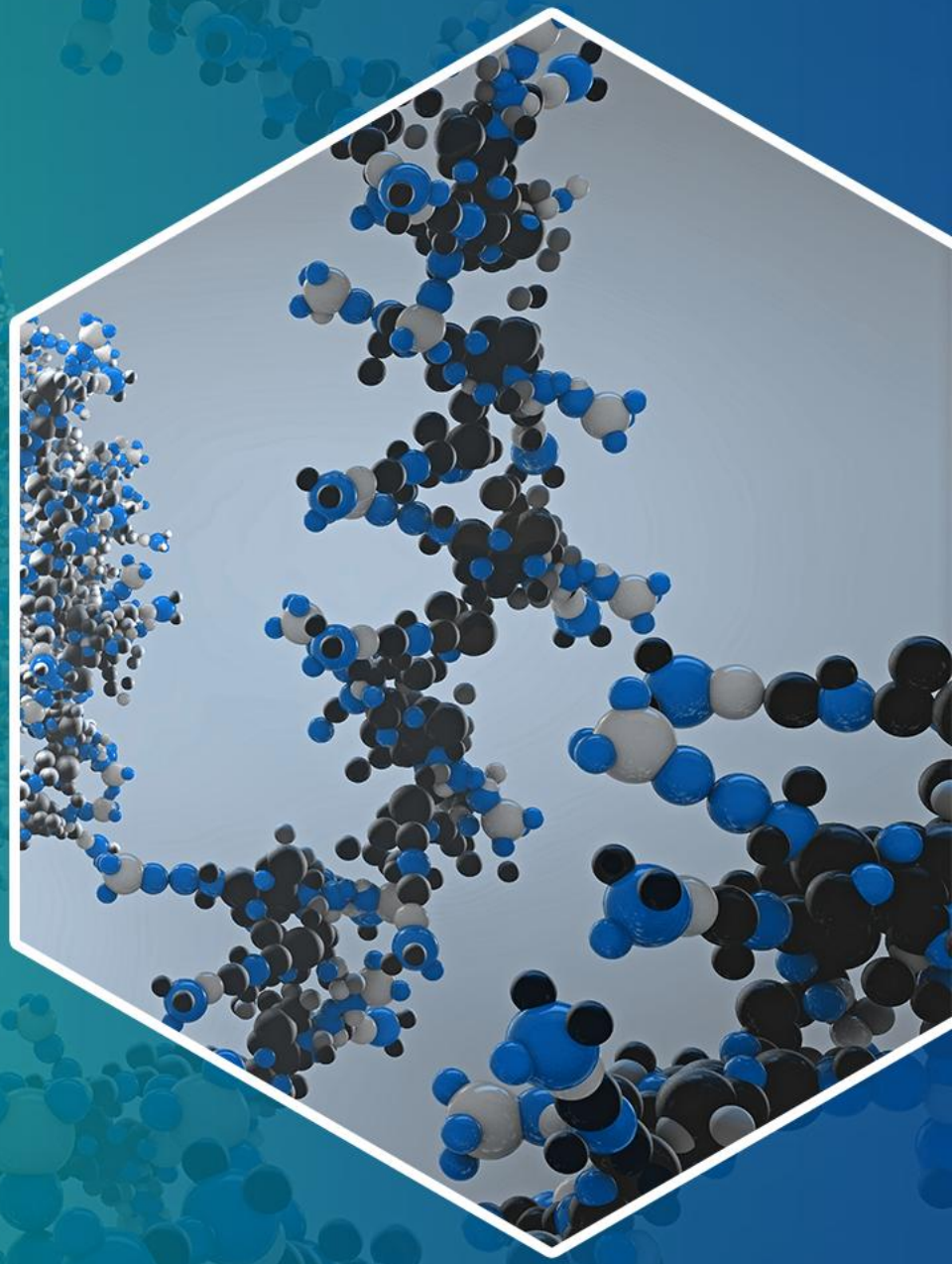


Understanding why trials fail is essential — but equally critical is knowing what it takes for trials to succeed. This graph shows the duration of all completed phase 1 and phase 1/2 trials investigating an ASO program that has since progressed to later phases of development and is still actively being developed, as these can be deemed as successes in the space.

So far, 16 ASO trials have been completed for a drug that then progressed further in clinical trial testing. It appears that trial duration varies widely depending on the disease investigated, with successful phase 1 trials in the ASO space having taken between 12 to 58 months to conclude. This outlines that rare monogenic disorders trials take less to run and generate results compared to complex neurodegenerative diseases.

*Note: at this stage, the sample size is quite small, this is solely for informative purposes

Deals and Companies

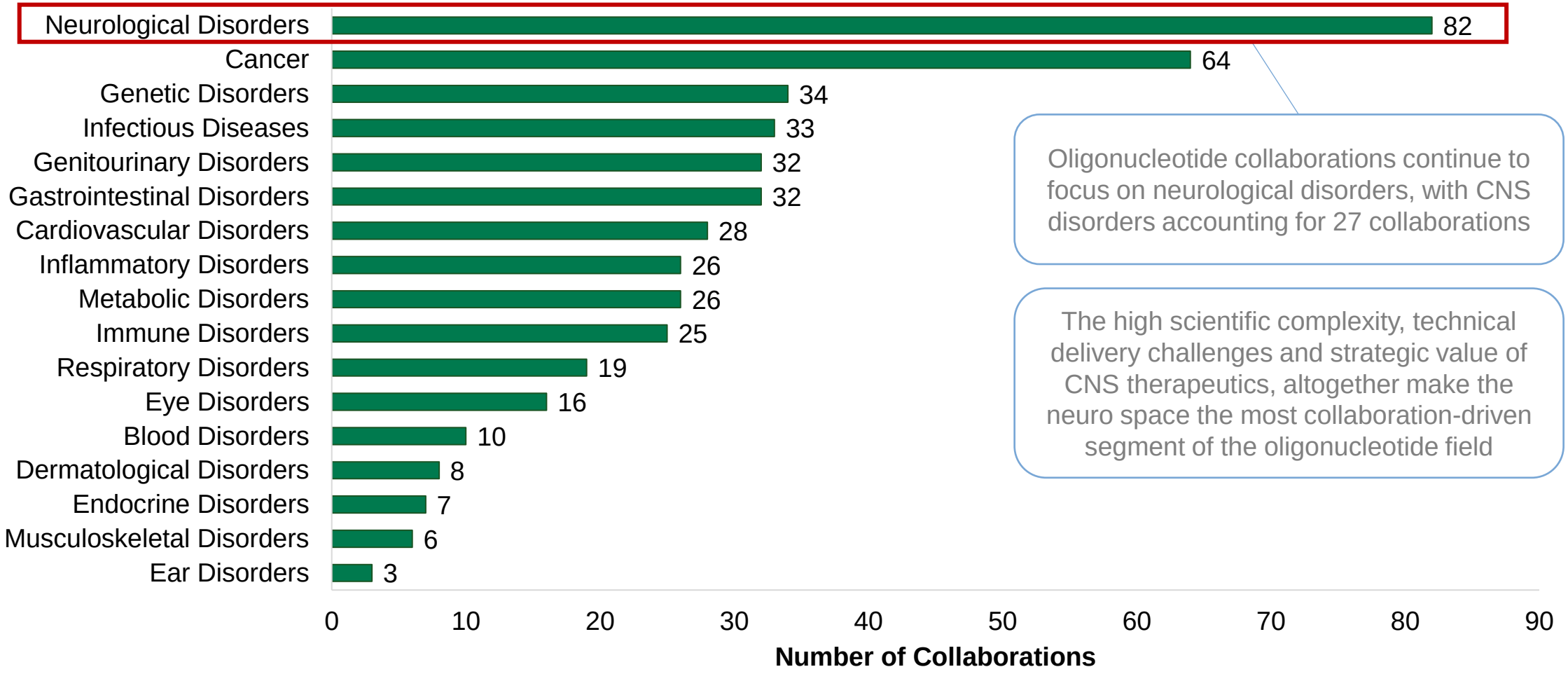


RNA Beacon byhansonwade

hansonwade

Collaborations by Disease

Diseases of Interest for Oligonucleotide Collaborations



Oligonucleotide collaborations continue to focus on neurological disorders, with CNS disorders accounting for 27 collaborations

The high scientific complexity, technical delivery challenges and strategic value of CNS therapeutics, altogether make the neuro space the most collaboration-driven segment of the oligonucleotide field

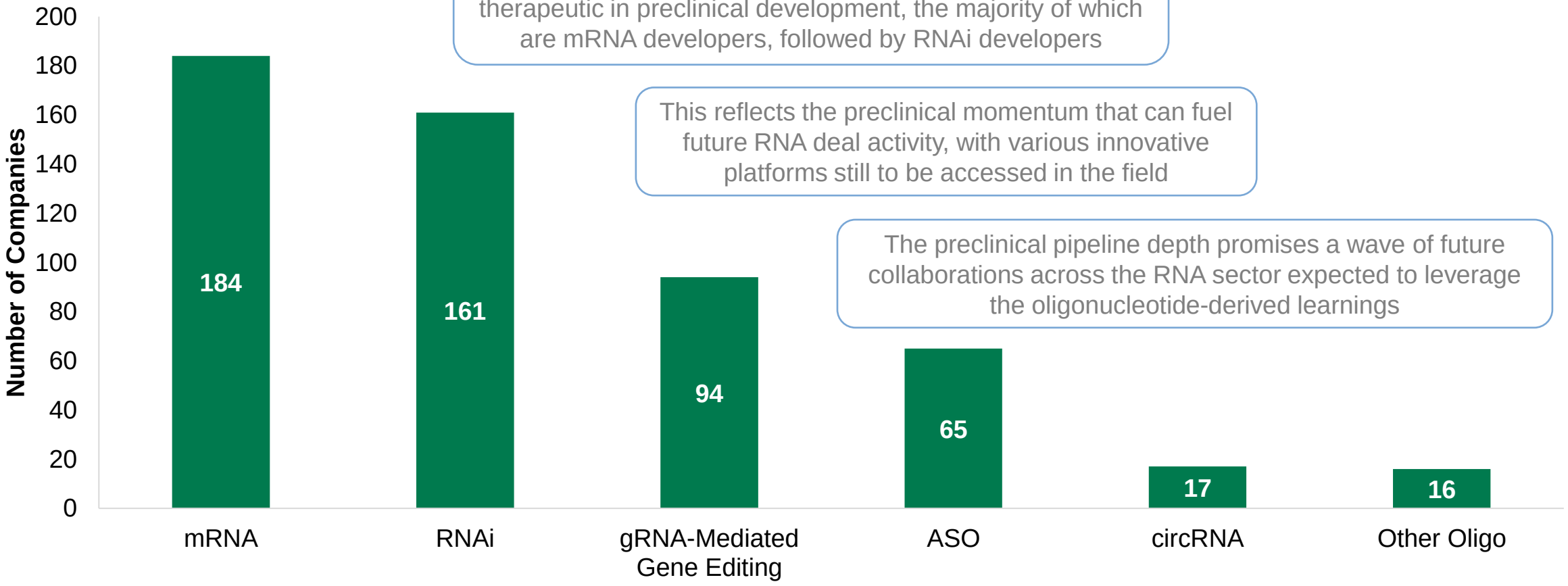
Preclinically Active Companies

Active Biopharma Companies in Preclinical Stages

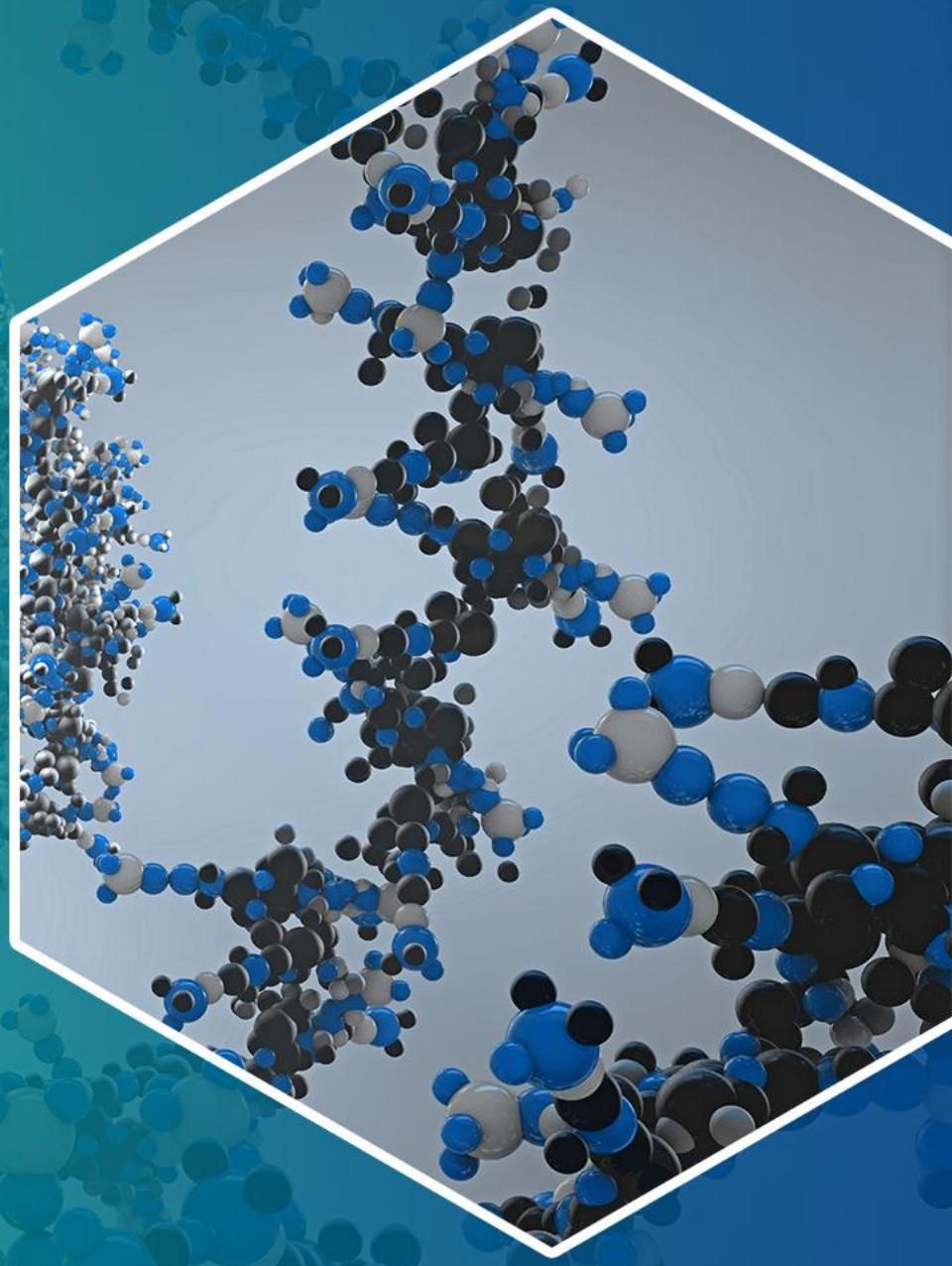
560+ active biopharma companies in the RNA space are yet to reach clinical stage, having at least one RNA-based therapeutic in preclinical development, the majority of which are mRNA developers, followed by RNAi developers

This reflects the preclinical momentum that can fuel future RNA deal activity, with various innovative platforms still to be accessed in the field

The preclinical pipeline depth promises a wave of future collaborations across the RNA sector expected to leverage the oligonucleotide-derived learnings



How We Help Oligonucleotide Development



RNA Beacon byhansonwade

hansonwade

How Beacon RNA Works

Content Sources

- Conference Abstracts & Posters
- Newspapers And Other Media Sites
- Government & Regulatory Orgs
- Scientific Journals And Publications
- Press Releases
- Patents
- Clinical Trial Registries
- Company Reports & Presentations

Human Curation

Ioana Panait
RNA Research Lead

All public data from 40,000+ sources curated by hand

24-48 hr window from data publication to curation & entry

Unique coverage of early-stage pipeline & preclinical data

Uncovering 30% more data from public domain sources

Drug Records

- Preclinical Data
- Platform Technology & Delivery Insights
- Regulatory Announcements
- Milestones & Development Timelines

Trial Records

- Clinical Readouts
- Study Design
- Arms
- Trial Locations
- Patient Data

Reports & Analysis

- Topical Landscape Overviews
- Conference Pre-Planners
- Post Conference Reports

Deals & Company Data

- Company Profiles
- Partnership & Licensing Deals
- Fundraising Activities
- Company Pipelines

Who Uses Beacon RNA

Oligonucleotide Drug Developers

- Pipeline Benchmarking
- Discovery Research
- Molecular Design
- Clinical Trial Design
- Fundraising & Competitive Positioning

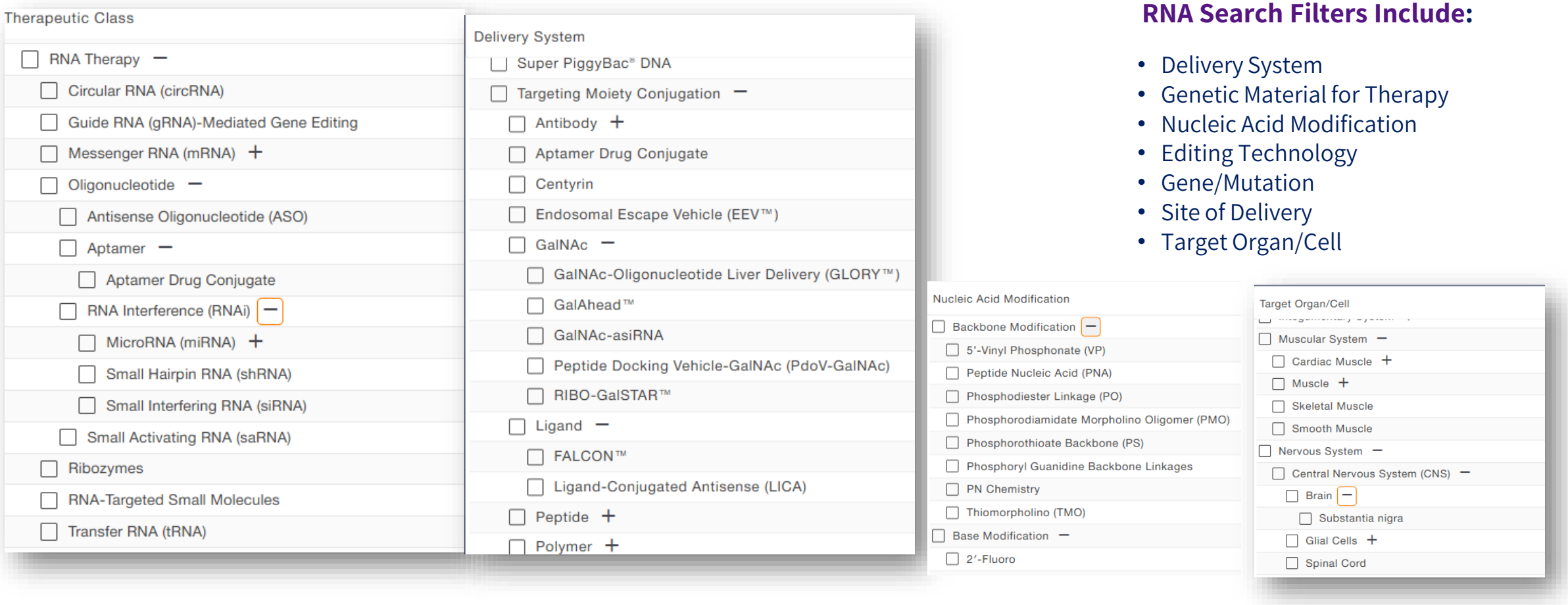
Oligonucleotide Solution Providers

- Identify Partnership Opportunities Early
- Understand Future Client Demand
- Support Technical Understanding of Complex Modalities

Academic Research Groups

- Identify New Oligonucleotide Technologies
- Understand Which Experiments Have Shown Success
- Identify Commercial Partners To Being Research to Patients

Beacon RNA Searching Filters



RNA Search Filters Include:

- Delivery System
- Genetic Material for Therapy
- Nucleic Acid Modification
- Editing Technology
- Gene/Mutation
- Site of Delivery
- Target Organ/Cell

Data Curation - Preclinical

Mivelsiran ☆

Active > Clinical | RNA | ADC | Neuroscience

Summary | Sequence and Mutation | Preclinical Data | CMC | Clinical Data | Pivotal Trials | Asset History | Milestones | Regulatory Announcements | Comme

Efficacy

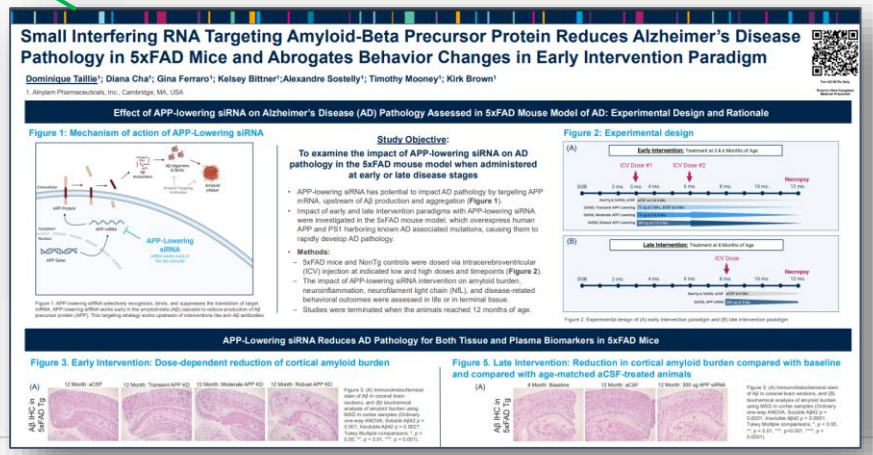
2025 AAIC Poster:

- Model: 5xFAD mice
- Treatment: 5xFAD mice and NonTg controls were dosed via intracerebroventricular (ICV) injection at indicated low and high doses
- Result:
  - APP-Lowering siRNA Reduces AD Pathology for Both Tissue and Plasma Biomarkers in 5xFAD Mice
    - Early Intervention: Dose-dependent reduction of cortical amyloid burden
    - Late Intervention: Reduction in cortical amyloid burden compared with baseline and compared with age-matched aCSF-treated animals
    - Early Intervention: Dose-dependent reduction in plasma NfL and cortical GFAP
    - Late Intervention: Reduction in plasma NfL and cortical GFAP
  - APP-Lowering siRNA Abrogates Behavior Changes in Early Intervention Paradigm
    - 5xFAD mice increase the time spent in the open arms of the EPM as disease pathology progresses
    - Increased time spent in the open arms is representative of abnormal behavior and decreased anxiety-like behavior
    - Early Intervention: Dose-dependent change in behavior phenotype with robust APP KD resulted in similar behavior to non-Tg animals at all timepoints
    - Late Intervention: No significant change in behavior phenotype at 12 months of age in siRNA-treated compared with age-matched aCSF-treated animals

Taillie D., et al. 2025 Alzheimer's Association International Conference Poster

2025 Corporate Presentation:

- Model: CVN mouse model
- Assay: Immunohistochemical analysis
- Treatment: 120ug single dose of APP siRNA by ICV
- Result:
  - Result showed APP mRNA Expression after single dose of siRNA
  - Showed significant reductions in Aβ parenchymal and vascular accumulation in the hippocampus compared with controls 3 and 6 months post-dose


Small Interfering RNA Targeting Amyloid-Beta Precursor Protein Reduces Alzheimer's Disease Pathology in 5xFAD Mice and Abrogates Behavior Changes in Early Intervention Paradigm

Dominique Taillie[1]; Diana Cha[1]; Gina Ferraro[1]; Kelsey Bittner[1];Alexandre Sostelly[1]; Timothy Mooney[1]; Kirk Brown[1]

1. Alnylam Pharmaceuticals, Inc., Cambridge, MA, USA

Effect of APP-lowering siRNA on Alzheimer's Disease (AD) Pathology Assessed in 5xFAD Mouse Model of AD: Experimental Design and Rationale

Figure 1: Mechanism of action of APP-Lowering siRNA

Study Objective:

To examine the impact of APP-lowering siRNA on AD pathology in the 5xFAD mouse model when administered at early or late disease stages

- APP-lowering siRNA has potential to impact AD pathology by targeting APP mRNA, upstream of Aβ production and aggregation (Figure 1).
- Impact of early and late intervention paradigms with APP-lowering siRNA were investigated in the 5xFAD mouse model, which overexpress human APP and PS1 harboring known AD associated mutations, causing them to rapidly develop AD pathology.
- Methods:
  - 5xFAD mice and NonTg controls were dosed via intracerebroventricular (ICV) injection at indicated low and high doses and timepoints (Figure 2).
  - The impact of APP-lowering siRNA intervention on amyloid burden, neuroinflammation, neurofilament light chain (NfL), and disease-related behavioral outcomes were assessed in life or in terminal tissue.
  - Studies were terminated when the animals reached 12 months of age.

Figure 2: Experimental design

APP-Lowering siRNA Reduces AD Pathology for Both Tissue and Plasma Biomarkers in 5xFAD Mice

Figure 3. Early Intervention: Dose-dependent reduction of cortical amyloid burden

Figure 5. Late Intervention: Reduction in cortical amyloid burden compared with baseline and compared with age-matched aCSF-treated animals

Preclinical Data Includes:

- In vitro & In Vivo Data
- Efficacy
- Cell Line Information
- Animal Models
- Dosing
- Toxicity
- Results

Clinical Results Data

Safety, Tolerability, Pharmacodynamic, Efficacy, and Pharmacokinetic Study of DYNE-251 in Participants With Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping (DELIVER)

Active not recruiting | ADC | RNA

Summary | Study | Inclusion/Exclusion | Trial Locations | Arms | Drug Dosing | Patients | Toxicity | Results | References | Commentary | Update History

General Explanation

2025 AAN:

- DYNE-251 drove dose-dependent increases in mean PMO muscle concentration and exon skipping, resulting in 3.22% and 3.72% of normal mean dystrophin in the 10 mg/kg and 20 mg/kg cohorts, respectively, at 6 months
- The mean corresponding muscle content-adjusted dystrophin levels were 7.64% and 8.72% of normal
- DYNE-251 led to improvements across multiple functional endpoints, including the North Star Ambulatory Assessment, Time to Rise from Floor, 10-Meter Walk/Run Test, and Stride Velocity 95th Centile
- Improvements were noted as early as 6 months in both the 10 mg/kg and 20 mg/kg cohorts; a continued effect through 12 months was observed in the 10 mg/kg cohort
- Subsequently, all participants in the 40 mg/kg cohorts were lowered to the 20 mg/kg dose level

2025 MDA Press Release:

- Meaningful and sustained improvements from baseline in multiple functional endpoints were observed in both the 20 mg/kg (selected registrational dose) and 10 mg/kg DYNE-251 Q4W cohortss, through 12 and 18 months, respectively
- Starting at the 6-month timepoint, the SV95C change from baseline observed in both the 10 mg/kg and 20 mg/kg cohorts of DELIVER exceeded the published proposed minimal clinically important difference
- SV95C is a digital objective outcome measure of ambulatory performance in patients
- As previously reported, DYNE-251 demonstrated unprecedented near-full length dystrophin expression as measured by Western blot for patients with DMD who are amenable to exon 51 skipping
- At the 6-month time point, patients treated with 20 mg/kg of DYNE-251 Q4W had a mean absolute dystrophin expression of 8.72% of normal (adjusted for muscle content)

2025 MDA Abstract:

- DYNE-251 drove dose-dependent increases in mean PMO muscle concentration and exon skipping, resulting in 3.22% and 3.72% of normal mean dystrophin in the 10 mg/kg and 20 mg/kg cohorts, respectively, at 6 months
- The mean corresponding muscle content-adjusted dystrophin levels were 7.64% and 8.72% of normal
- DYNE-251 led to improvements across multiple functional endpoints, including the North Star Ambulatory Assessment, Time to Rise from Floor, 10-Meter Walk/Run Test, and Stride Velocity 95th Centile
- Improvements were noted as of 6 months in the 10 mg/kg and 20 mg/kg cohorts, with a continued effect through 12 months in the 10 mg/kg cohort

2024 WMS:

- DYNE-251 showed mean absolute dystrophin level, measured by Western blot, increased from 0.60% at baseline to 0.88% of normal at 6 months, and the mean level of dystrophin positive fibers (PDPF) increased from 2.4% at baseline to 22.2% at 6 months.

Company Data – Deals & Pipeline

Arrowhead Pharmaceuticals and Novartis Enter into a Global License and Collaboration Agreement

September 2, 2025

PDF Version

- Upon closing, Arrowhead will receive an upfront payment of $200 million

- Novartis will receive an exclusive worldwide license to ARO-SNCA, Arrowhead's preclinical stage siRNA therapy for the treatment of synucleinopathies, such as Parkinson's Disease, plus additional collaboration targets

PASADENA, Calif.--(BUSINESS WIRE)--Sep. 2, 2025-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced a global licensing and collaboration agreement with Novartis for ARO-SNCA, Arrowhead's preclinical stage siRNA therapy against alpha-synuclein for the treatment of synucleinopathies, such as Parkinson's Disease, and for other additional collaboration targets that will utilize Arrowhead's proprietary Targeted RNAi Molecule (TRiM™) platform. Upon closing, Arrowhead will receive $200 million as an upfront payment and is eligible to receive up to $2 billion in potential milestone payments plus royalties on commercial sales.

Filters: Arrowhead Pharmaceuticals

esults: 34 Results per page: 100 1 of 1 Save Search Manage Columns Export Copy

Dataset	Name	Developers	Drug Status	Highest Phase of Developm	Disease Indication	Drug Targets	Genetic Material for Therapy	Delivery System	Target Organ/Cell	Nucleic A
ADC, RNA	Olpasiran AMG 890, ARO-LPA	Amgen Inc, Arrowhead Pharmaceuticals, Royalty Pharma	Active > Clinical	3	Atherosclerosis, Atherosclerotic Cardiovascular Disease…	LPA ASGPR	siRNA (small interfering RNA)	GalNAc, Targeted RNAi Molecule…	Liver	Undisclos
ADC, RNA	Zodasiran ARO-ANG3, VSA003	Arrowhead Pharmaceuticals, Visirna Therapeutics	Active > Clinical	3	Dyslipidemia, Familial Hypercholesterolemia, Healthy…	ANGPTL3 ASGPR	siRNA (small interfering RNA)	Targeted RNAi Molecule (TRiM™)…	Liver	Undisclos
ADC, RNA	Plozasiran ADS-005, ARO-APOC3, VSA001	Arrowhead Pharmaceuticals, Sanofi, Visirna Therapeutics	Active > Clinical	3	Acute Pancreatitis, Dyslipidemia, Familial Chylomicronemia…	APOC3 ASGPR	siRNA (small interfering RNA)	GalNAc, Targeted RNAi Molecule…	Liver	2′-Fluoro,
ADC, RNA	Fazirsiran ADS-001, ARO-AAT, TAK-999	Arrowhead Pharmaceuticals, Takeda	Active > Clinical	3	Alpha-1 Antitrypsin Deficiency, Healthy Volunteers, Hepatic…	SERPINA 1 ASGPR	siRNA (small interfering RNA)	GalNAc, Targeted RNAi Molecule…	Liver	2′-O-Meth Phosphor
ADC, RNA	ARO-HSD GSK4532990, VSA006	Arrowhead Pharmaceuticals, GlaxoSmithKline, Visirna Therapeutics	Active > Clinical	2	Acute Chronic Liver Diseases, Alcoholic Liver Disease,…	HSD17B1 3 ASGPR	siRNA (small interfering RNA)	GalNAc, Targeted RNAi Molecule…	Liver	Undisclos

Arrowhead Pharmaceuticals

Active | ADC | Autoimmune | RNA | Oncology | Gene Therapy

Summary | Deals | Pipeline | References | Update History

Deals

Arrowhead Pharmaceuticals and Novartis Enter into a Global License and Collaboration Agreement

Dataset:	RNA
Deal Type:	Partnership & Collaboration, Licensing Agreement
Asset:	Drug (ARO-SNCA)
Companies:	Novartis Pharmaceuticals AG
Potential Total Deal Value:	USD 2.2bn + royalties
Date:	02 Sep 2025

Arrowhead Pharmaceuticals Announces Global License and Collaboration Agreement with Sarepta Therapeutics for Multiple Clinical and Preclinical Programs

Dataset:	ADC, RNA, Autoimmune
Deal Type:	Licensing Agreement, Partnership & Collaboration
Asset:	Drug (ARO-DUX4 (SRP-1001)), Drug (ARO-DM1 (SRP-1003)), Drug (ARO-MMP7), Enabling Technology, Drug (ARO-ATXN2 (SRP-1004)), Drug (ARO-HTT), Drug (ARO-ATXN1), Drug (ARO-ATXN3)
Companies:	Sarepta Therapeutics, Inc.
Potential Total Deal Value:	USD 11.4bn + royalties
Date:	25 Nov 2024

Arrowhead Sells Royalty Interest in Olpasiran to Royalty Pharma for $250 Million and Future Milestones

Dataset:	RNA, ADC
Deal Type:	Licensing Agreement
Asset:	Drug (Olpasiran (AMG 890, ARO-LPA))
Companies:	Royalty Pharma
Potential Total Deal Value:	USD 410m + royalties
Date:	09 Nov 2022

Arrowhead Pharmaceuticals and Vivo Capital Launch Joint Venture Aimed at Greater China Market

Dataset:	RNA, ADC, Autoimmune
Deal Type:	Joint Venture
Companies:	Vivo Capital, Visirna Therapeutics
Potential Total Deal Value:	Not Available
Date:	25 Apr 2022

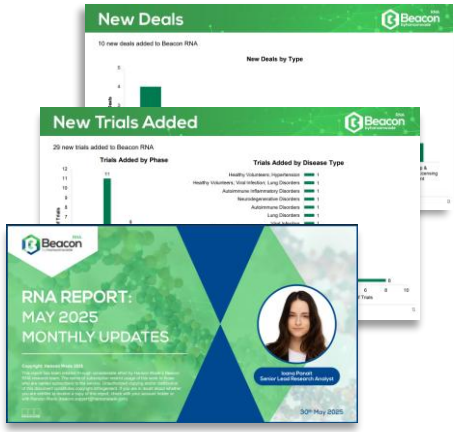
Arrowhead exclusively licenses ARO-HSD to GSK for all regions outside Greater China

Dataset:	ADC, RNA
Deal Type:	Licensing Agreement
Asset:	Drug (ARO-HSD (GSK4532990, VSA006))
Companies:	GlaxoSmithKline
Potential Total Deal Value:	USD 1bn + royalties
Date:	22 Nov 2021

Arrowhead Entered into a Collaboration and License Agreement with Horizon Therapeutics Ireland DAC ("Horizon") For RNAi Therapeutic

Dataset:	ADC, RNA
Deal Type:	Licensing Agreement, Partnership & Collaboration
Asset:	Drug (ARO-XDH (HZN-457))
Companies:	Horizon Therapeutics
Potential Total Deal Value:	USD 700m + royalties
Date:	18 Jun 2021

Reports & Analysis



Monthly Analysis

Insight & Infographics

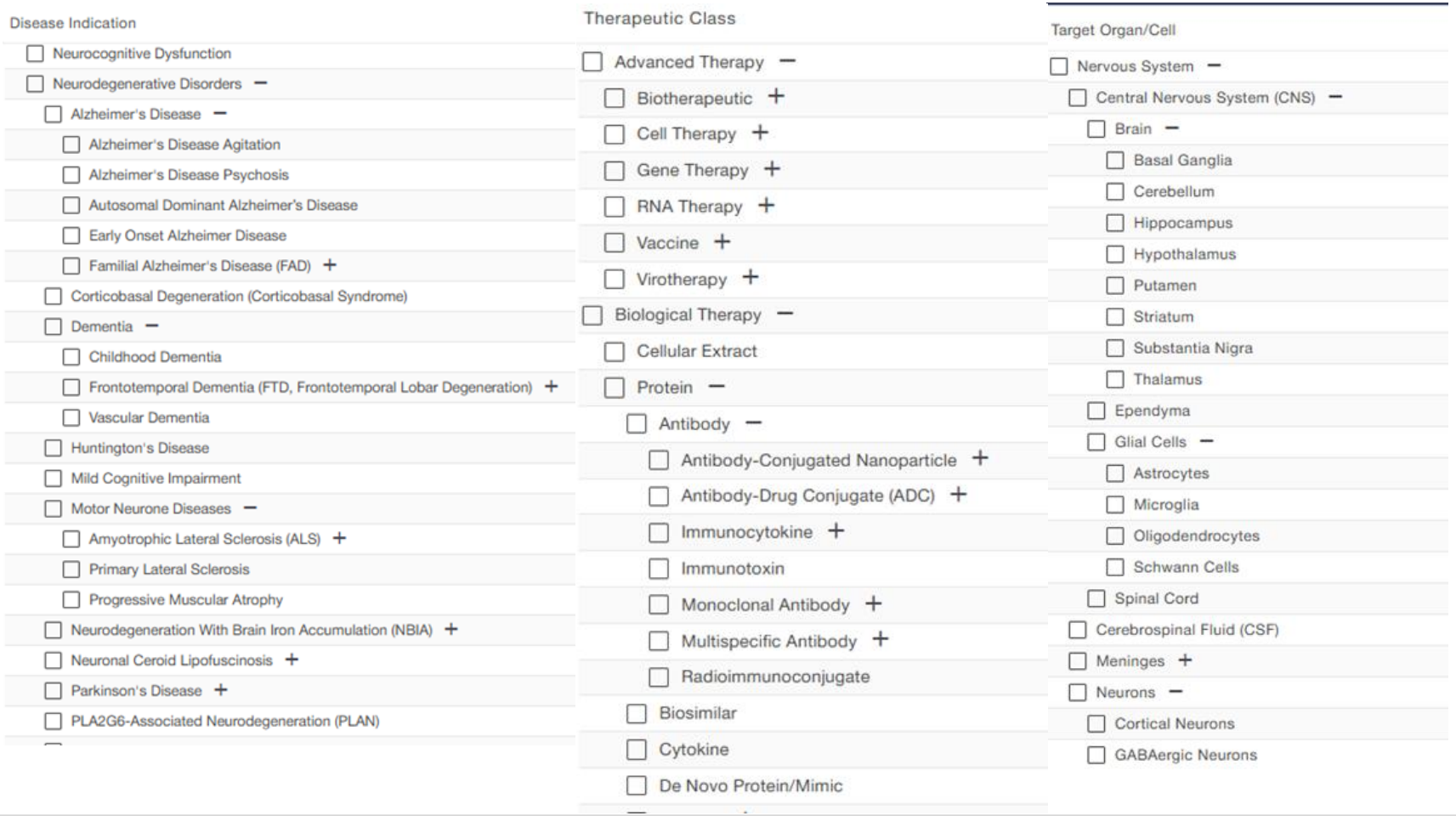
Intelligence & White Papers

Conference Reports

New Beacon Neuroscience Dataset

Neuroscience Beacon byhansonwade

- **7,000+ Neuroscience Drugs**
- **8,000+ Neuroscience Trials**
- **All Drug Modalities**
- **40,000+ Sources of Curated Preclinical & Clinical Data**
- **Unique Searching Ontologies**



Join Us At OTS Annual Meeting!

https://www.2025oligomeeting.com/

Ioana Panait
RNA Senior Research Lead
Ioana.panait@hansonwade.com

Matty Slingsby
Commercial Manager - RNA
Matty.slingsby@hansonwade.com

Questions?

Schedule a demonstration here

Contact the Team

Ioana Panait
RNA Senior Research Lead
Ioana.panait@hansonwade.com

Matty Slingsby
Commercial Manager - RNA
Matty.slingsby@hansonwade.com

Thank you

MAKE FASTER, BETTER DRUG DEVELOPMENT DECISIONS

beacon.support@hansonwade.com
www.beacon-intelligence.com

hansonwade

4th September 2025