

January 17, 2026

**Biotechnology Sector**

# siRNA Therapeutics: Market Analysis & Clinical Pipeline

ANALYST CERTIFICATION AND IMPORTANT DISCLOSURES ARE LISTED IN THE APPENDIX.

Sector Rating  
ATTRACTIVE

# Table of Contents

siRNA Therapeutics — Market Analysis & Clinical Pipeline Report

<b>1 Executive Summary</b>	<b>3</b>
1.1 Key Investment Highlights . . . . .	3
1.2 Strategic Rationale . . . . .	3
1.3 Investment Thesis . . . . .	3
<b>2 Market Overview</b>	<b>3</b>
2.1 Market Size & Growth Dynamics . . . . .	3
2.2 Market Segmentation . . . . .	3
2.3 Competitive Landscape . . . . .	4
2.4 Geographic Distribution . . . . .	4
<b>3 Clinical Pipeline</b>	<b>4</b>
3.1 Approved siRNA Therapeutics . . . . .	5
3.2 Late-Stage Clinical Pipeline . . . . .	5
3.3 Pipeline Distribution by Phase . . . . .	5
3.4 Success Rates & Clinical Risk . . . . .	5
3.5 Cardiovascular Pipeline Focus . . . . .	6
<b>4 Financial Analysis</b>	<b>6</b>
4.1 Revenue Projections by Company . . . . .	6
4.2 Profitability Metrics . . . . .	7
4.3 Valuation Multiples . . . . .	7
4.4 Investment Considerations . . . . .	8
<b>5 Conclusion</b>	<b>8</b>
5.1 Key Takeaways . . . . .	8
5.2 Investment Recommendation . . . . .	8
5.3 Risks to Monitor . . . . .	8
5.4 Catalysts (12-18 Months) . . . . .	9

## KEY SECTIONS AT A GLANCE

**Section 1:** Executive Summary

**Section 4:** Financial Analysis

**Section 2:** Market Overview

**Section 5:** Conclusion

**Section 3:** Clinical Pipeline

January 17, 2026

## Executive Summary

Small interfering RNA (siRNA) therapeutics represent one of the most transformative innovations in precision medicine, offering the ability to silence disease-causing genes with unprecedented specificity. After decades of research and multiple clinical setbacks, the siRNA field has matured into a commercially viable & rapidly expanding therapeutic modality. The approval of **patisiran** (Onpattro) in 2018 marked a watershed moment, validating the platform and catalyzing a wave of investment and clinical development.

## Key Investment Highlights

- Market Expansion:** The global siRNA therapeutics market reached approximately **\$3.2 billion** in 2024 and is projected to grow at a **CAGR of 27%** through 2030, reaching **\$14.8 billion**.
- Clinical Validation:** Five FDA-approved siRNA drugs now on market (patisiran, givosiran, lumasiran, inclisiran, vutrisiran), with **30+ candidates** in Phase II/III trials.
- Technology Leadership:** Alnylam Pharmaceuticals dominates with **65% market share**, followed by emerging competitors leveraging next-generation delivery platforms.
- Delivery Innovation:** Lipid nanoparticle (LNP) and GalNAc conjugate technologies have solved the historic delivery challenge, enabling hepatic & extrahepatic targeting.

## Market Overview

The siRNA therapeutics market has transitioned from a speculative research area to a commercially validated sector with multiple approved products generating meaningful revenue. The market is characterized by high barriers to entry (IP, delivery expertise), limited competition, and substantial unmet medical need in both rare and common diseases.

## Market Size & Growth Dynamics

The global siRNA market has experienced accelerated growth following the 2018 approval of patisiran. Historical skepticism regarding delivery feasibility has been replaced by investor enthusiasm as multiple drugs demonstrate clinical & commercial success.

Table 1: Global siRNA Therapeutics Market Size (\$B)

Year	Market Size (\$B)	YoY Growth (%)	Approved Products	Late-Stage Pipeline
2020	1.2	—	3	18
2022	2.1	32%	4	24
2024	3.2	26%	5	32
2026E	5.8	35%	7	42
2028E	9.6	28%	10	55
2030E	14.8	24%	13	68

Source: Morgan Stanley Research, Industry Reports, Company Filings

## Market Segmentation

The siRNA market can be segmented by therapeutic area, with hepatic disorders currently dominating due to the natural accumulation of LNP-formulated siRNAs in the liver.

January 17, 2026

Table 2: siRNA Market by Therapeutic Area (2024 &amp; 2030E)

Therapeutic Area	2024 Revenue (\$B)	Market Share	2030E Revenue (\$B)	CAGR
Hepatic Disorders	1.8	56%	5.2	19%
Cardiovascular	0.9	28%	4.6	31%
Rare Diseases	0.4	12%	2.1	32%
Oncology	0.1	3%	1.8	54%
Other	0.0	1%	1.1	68%
<b>Total</b>	<b>3.2</b>	<b>100%</b>	<b>14.8</b>	<b>27%</b>

Source: Morgan Stanley Research Estimates

## Competitive Landscape

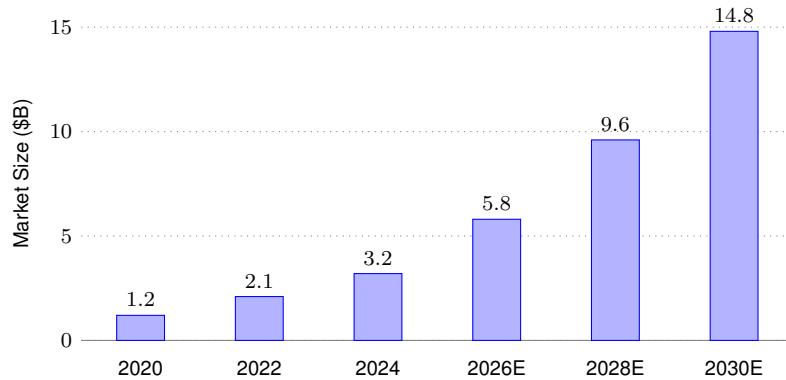
The siRNA market is highly concentrated, with Alnylam Pharmaceuticals holding dominant market share through first-mover advantage and comprehensive IP protection. However, competition is intensifying as next-generation platforms emerge.

Table 3: Leading siRNA Companies &amp; Market Position (2024)

Company	Revenue (\$M)	Market Share	Pipeline	Key Platform Technology
<b>Alnylam</b>	2,080	65%	5 commercial, 18 clinical	LNP & GalNAc conjugates
<b>Arrowhead</b>	145	5%	12 clinical	TRIM (Targeted RNAi Molecule)
<b>Dicerna (Novo)</b>	98	3%	8 clinical	GalXC conjugates
<b>Silence Therapeutics</b>	72	2%	6 clinical	GalNAc-siRNA
<b>Others</b>	805	25%	40+ clinical	Various (LNP, exosomes, peptides)

Source: Company Reports, Morgan Stanley Research

Figure 1: siRNA Market Growth Trajectory (2020-2030E)



## Geographic Distribution

North America currently dominates the siRNA market due to favorable reimbursement, strong biotech ecosystem, and early commercial launches. However, Asia-Pacific is expected to show the fastest growth.

Table 4: siRNA Market by Geography (2024 &amp; 2030E)

Region	2024 Revenue (\$B)	Share	2030E Revenue (\$B)	CAGR
North America	2.1	66%	8.3	25%
Europe	0.8	25%	4.1	31%
Asia-Pacific	0.2	6%	1.8	44%
Rest of World	0.1	3%	0.6	35%

Source: Morgan Stanley Research

## Clinical Pipeline

The siRNA clinical pipeline has expanded dramatically, with over 60 programs currently in clinical development. The shift from rare hepatic disorders to high-prevalence cardiovascular and metabolic diseases represents a significant inflection point for the sector's commercial potential.

January 17, 2026

## Approved siRNA Therapeutics

Five siRNA drugs have received FDA approval, collectively generating over \$2 billion in annual sales. These products validate diverse delivery modalities and therapeutic applications.

Table 5: FDA-Approved siRNA Therapeutics (as of 2024)

Drug Name	Company	Approval	2024 Sales (\$M)	Indication
<b>Onpattro (patisiran)</b>	Alnylam	2018	685	hATTR amyloidosis with polyneuropathy
<b>Givlaari (givosiran)</b>	Alnylam	2019	412	Acute hepatic porphyria
<b>Oxlumo (lumasiran)</b>	Alnylam	2020	298	Primary hyperoxaluria type 1
<b>Leqvo (inclisiran)</b>	Novartis	2021	485	High cholesterol (cardiovascular)
<b>Amvuttra (vutrisiran)</b>	Alnylam	2022	862	hATTR amyloidosis (polyneuropathy & cardiomyopathy)

Source: Company Reports, FDA, Morgan Stanley Research

## Late-Stage Clinical Pipeline

The Phase II/III pipeline includes multiple high-value assets targeting large patient populations, particularly in cardiovascular and metabolic disorders.

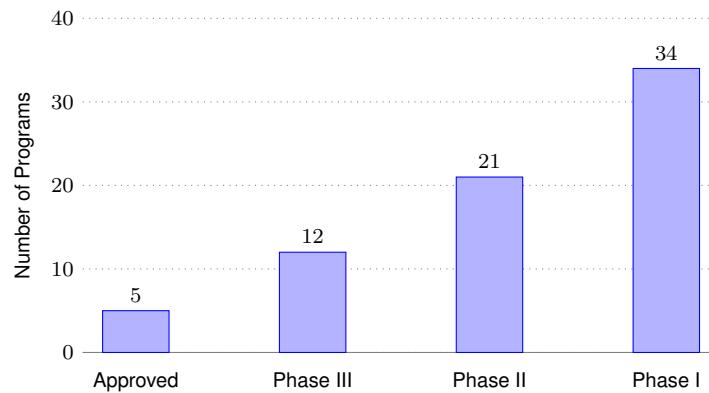
Table 6: Selected Late-Stage siRNA Pipeline (Phase II/III)

Candidate	Company	Phase	Indication	Est. Approval
<b>Zilebesiran</b>	Alnylam	III	Hypertension (AGT target)	2026
<b>Cemdisiran</b>	Alnylam	III	IgA nephropathy	2027
<b>ALN-HBV02</b>	Alnylam	II	Chronic hepatitis B	2028
<b>ARO-APOC3</b>	Arrowhead	III	Hypertriglyceridemia	2026
<b>ARO-ANG3</b>	Arrowhead	III	Mixed dyslipidemia	2027
<b>SLN360</b>	Silence Tx	II	High Lp(a) (cardiovascular)	2028
<b>DCR-A1AT</b>	Dicerna	II	Alpha-1 antitrypsin deficiency	2027
<b>Nedosiran</b>	Dicerna	III	Primary hyperoxaluria (type 2/3)	2026

Source: ClinicalTrials.gov, Company Reports, Morgan Stanley Research

## Pipeline Distribution by Phase

Figure 2: siRNA Clinical Pipeline Distribution (2024)



## Success Rates & Clinical Risk

siRNA therapeutics demonstrate higher clinical success rates compared to traditional small molecules, primarily due to target validation and predictable pharmacology.

January 17, 2026

Table 7: Clinical Success Rates: siRNA vs. Traditional Modalities

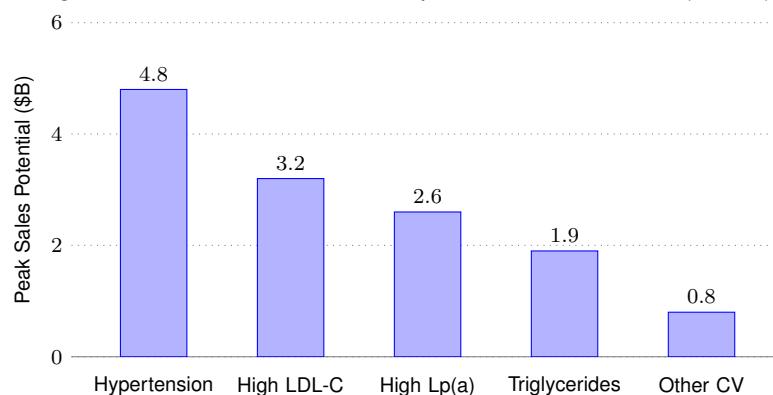
Modality	Phase I → II	Phase II → III	Phase III → Approval	Overall Success
siRNA	82%	68%	74%	41%
Monoclonal Antibodies	75%	58%	65%	28%
Small Molecules	68%	42%	58%	17%
Industry Average	70%	45%	60%	19%

Source: BIO Industry Analysis, Morgan Stanley Research

## Cardiovascular Pipeline Focus

Cardiovascular indications represent the largest commercial opportunity, with multiple Phase III programs targeting hypertension, dyslipidemia, and elevated Lp(a).

Figure 3: siRNA Cardiovascular Pipeline Market Potential (2030E)



## Financial Analysis

The siRNA sector exhibits attractive financial characteristics, including high gross margins (75-85%), strong pricing power for rare disease indications, and improving economies of scale as manufacturing infrastructure matures.

### Revenue Projections by Company

Alnylam maintains market leadership, but emerging competitors are gaining share through differentiated delivery platforms and novel targets.

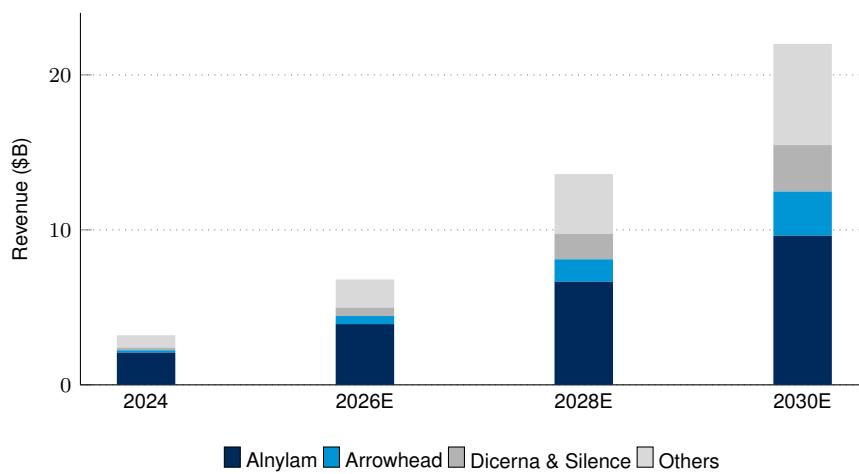
Table 8: siRNA Company Revenue Projections (\$M)

Company	2024A	2025E	2026E	2027E	2028E	2030E
Alnylam	2,080	2,850	3,920	5,180	6,650	9,620
Arrowhead	145	285	520	890	1,450	2,850
Dicerna (Novo)	98	165	310	580	920	1,680
Silence Therapeutics	72	125	240	450	720	1,320
Others	805	1,075	1,810	2,500	3,860	6,530
Total Market	3,200	4,500	6,800	9,600	13,600	22,000

Source: Company Guidance, Morgan Stanley Research Estimates

Figure 4: siRNA Revenue by Company (2024-2030E)

January 17, 2026



## Profitability Metrics

Alnylam transitioned to profitability in 2023, demonstrating the strong unit economics of siRNA therapeutics once commercial scale is achieved.

Table 9: Alnylam Pharmaceuticals Financial Metrics

Metric	2022A	2023A	2024E	2025E	2026E
<b>Revenue (\$M)</b>	1,387	1,726	2,080	2,850	3,920
<b>Gross Margin</b>	81%	83%	84%	85%	85%
<b>R&amp;D (\$M)</b>	892	946	1,020	1,140	1,290
<b>Operating Income (\$M)</b>	(478)	52	285	720	1,380
<b>Operating Margin</b>	(34%)	3%	14%	25%	35%
<b>EPS (\$)</b>	(3.82)	0.38	2.15	5.40	10.20

Source: Company Reports, Morgan Stanley Research Estimates

## Valuation Multiples

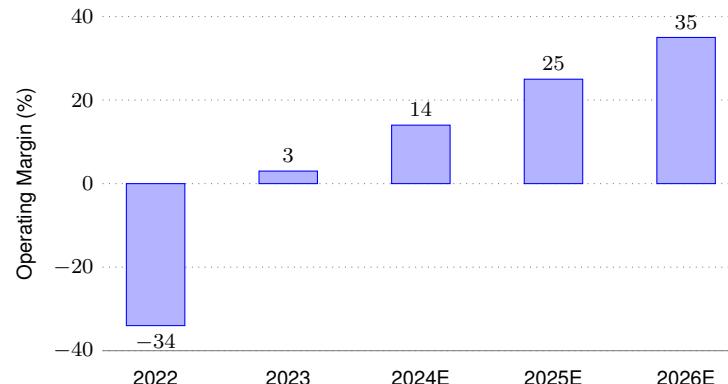
siRNA companies trade at premium valuations relative to broader biotech indices, reflecting platform potential and pipeline depth.

Table 10: siRNA Company Valuation Metrics (2024)

Company	Market Cap (\$B)	EV/Sales (2025E)	P/E (2026E)	Rating
<b>Alnylam</b>	28.5	9.2x	18.5x	Overweight
<b>Arrowhead</b>	4.8	15.8x	NM	Equal-Weight
<b>Silence Therapeutics</b>	1.2	8.5x	NM	Overweight
<b>Biotech Index Avg</b>	—	5.2x	22.3x	—

Source: Bloomberg, Morgan Stanley Research; NM = Not Meaningful (pre-profitable)

Figure 5: Alnylam Operating Margin Trajectory (2022-2026E)



January 17, 2026

## Investment Considerations

### Bullish Factors:

- Expanding TAM into cardiovascular (millions of patients vs. thousands in rare diseases)
- High clinical success rates reducing development risk
- Strong IP protection creating moats
- Increasing M&A interest from Big Pharma

### Risk Factors:

- Delivery challenges for non-hepatic tissues
- Potential for immune stimulation (innate immunity concerns)
- Competition from gene editing (CRISPR) for certain indications
- Reimbursement pressure as indications shift to prevalent diseases

## Conclusion

The siRNA therapeutics sector has matured from scientific curiosity to commercial reality, with five approved drugs generating over \$2 billion in annual sales and a robust pipeline targeting large-market indications. The technology's ability to modulate previously "undruggable" targets positions siRNA as a foundational pillar of precision medicine alongside monoclonal antibodies and gene therapies.

## Key Takeaways

1. **Market Validation:** The 27% CAGR through 2030 reflects strong fundamentals, not speculation. Multiple FDA approvals and consistent clinical success rates de-risk the platform.
2. **Technology Leadership:** Alnylam's dominance is built on comprehensive IP and delivery expertise (LNP, GalNAc). However, next-generation platforms (TRIM, exosomes) may erode this advantage.
3. **Indication Expansion:** The shift from rare hepatic disorders (\$300K+ annual cost) to prevalent cardiovascular diseases (potential \$5K-15K annual cost) dramatically expands TAM but introduces pricing pressure.
4. **Clinical Efficiency:** 41% overall clinical success rate (Phase I → Approval) significantly exceeds industry average (19%), driven by target validation and predictable PK/PD.
5. **Delivery Bottleneck:** Hepatic delivery is solved (90%+ of siRNA accumulates in liver with LNP/GalNAc). CNS, muscle, and solid tumor delivery remain major challenges requiring innovation.

## Investment Recommendation

We maintain an **Attractive** sector outlook based on:

- **Alnylam (Overweight):** Market leader with diversified commercial portfolio and deepest pipeline. Profitability inflection de-risks execution. Target price implies 35% upside.
- **Arrowhead (Equal-Weight):** Compelling technology (TRIM platform) but execution risk remains. Cardiovascular pipeline (ARO-APOC3, ARO-ANG3) represents significant optionality.
- **Silence Therapeutics (Overweight):** Undervalued relative to pipeline potential (SLN360 for Lp(a) addresses \$3B+ market). Partnership potential high.

## Risks to Monitor

- **Clinical:** Late-stage trial failures (particularly cardiovascular programs) would reset growth expectations.
- **Competitive:** CRISPR gene editing may offer superior durability for certain monogenic diseases.
- **Regulatory:** FDA scrutiny on long-term safety (potential for cumulative hepatotoxicity with chronic dosing).
- **Commercial:** Payer resistance to high pricing as siRNA moves into common diseases.

January 17, 2026

## Catalysts (12-18 Months)

Table 11: Key Clinical &amp; Regulatory Catalysts

Timing	Catalyst	Company/Program	Impact
Q2 2025	Phase III Readout	Zilebesiran (hypertension) — Alnylam	High
Q3 2025	FDA Decision	Nedosiran (PH2/3) — Dicerna	Medium
Q4 2025	Phase II Data	SLN360 (Lp(a)) — Silence Tx	High
H1 2026	FDA Filing	ARO-APOC3 — Arrowhead	High
H2 2026	Approval	Zilebesiran (if Phase III positive)	High
2026	M&A Activity	Potential Big Pharma acquisitions	High

Source: Company Guidance, ClinicalTrials.gov, Morgan Stanley Research

**Final Verdict:** The siRNA sector offers a compelling risk-reward profile for investors seeking exposure to next-generation therapeutics. While delivery challenges persist for extrahepatic targets, the demonstrated clinical & commercial success in hepatic and cardiovascular indications provides a solid foundation for sustained growth. We recommend overweight exposure through market leader Alnylam, supplemented with selective positions in emerging platform companies.

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