



Tracking the healthcare innovation

Free Insights

Antisense Oligonucleotide Therapeutics Market, 2021-2030

Target Indication (Duchenne Muscular Atrophy, Spinal Muscular Atrophy, Familial Chylomicronemia Syndrome and others), Type of Therapy (Monotherapy and Combination), Type of Molecule (DNA Molecule and RNA Molecule) Type of Generation (First, Second and Third) and Key Geographies (North America, Europe, Asia- Pacific and Rest of the World): Industry Trends and Global Forecasts

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Context

Advances in antisense oligonucleotide chemistry have enabled the development and synthesis of specialized lead molecules, having better cell targeting capabilities and improved safety profiles, for the treatment of a wide range of disease indications



Historical Evolution of Antisense Oligonucleotides

1978

Zamecnik and Stephenson used an oligonucleotide to inhibit Rous sarcoma virus replication

1998

The first antisense oligonucleotide-based therapy, Vitravene™, was approved in the US

2020

FDA approved VILTEPSO™ for the treatment of Duchenne muscular dystrophy

Since the identification of their therapeutic potential, antisense oligonucleotides have revolutionized the therapy landscape, permitting researchers to develop interventions against undruggable targets as well



Benefits of Antisense Oligonucleotide Therapeutics

Genetic Level Specificity

Enables the treatment of diseases characterized by both monogenic and polygenic errors

Advances in Drug Synthesis

Optimization of the ASO using gapmers has resulted in safety and stability-related improvements

Lasting Therapeutic Effect

By mediating changes at the gene level, these interventions offer prolonged clinical benefits

A dramatic increase in gene sequence information has enabled significant advances in the discovery and development of antisense therapeutics that are capable of altering the levels of expression of virtually any gene



Recent and Upcoming Developments

200+ Product Candidates

Marketed or under evaluation in the clinical / preclinical stages of development

60+ Partnerships

Have been inked amongst various stakeholders, between 2016 and 2020

USD Million 300+

Has been awarded in the form of grants, to finance the R&D efforts in this field

Driven by encouraging clinical trial results and the financial assistance of public funding institutes, we anticipate the antisense oligonucleotides market to grow at a healthy pace in the foreseen future

Project Objectives

Roots Analysis has done a detailed study on the **Antisense Oligonucleotide Therapeutics Market, 2021-2030**, covering key aspects of the industry and identifying potential future growth opportunities

Market Landscape



A detailed assessment of the current market landscape, featuring a comprehensive list of more than 200 therapies and 30+ companies along with analyses based on a number of parameters¹

Company Profiles



Elaborate profiles of key players, with each profile featuring a brief overview of the company, its financial information (if available), drug portfolio, recent developments and an informed future outlook

Clinical Trial Analysis



An in-depth analysis of completed, ongoing and planned clinical studies of various therapies, based on trial status, year of registration, trial phase, key indications, enrolled patient population and regional distribution of trials

Grant Analysis



A detailed analysis of more than 380 grants that have been awarded to support research projects related to antisense oligonucleotide therapeutics between 2017 and 2020, across multiple parameters²

Recent Collaborations



A review of the various partnerships and collaborations pertaining to antisense oligonucleotide therapeutics, featuring a detailed analysis based on various parameters, such as year of partnership, type of partnership and geographical landscape of the collaborating companies

Market Forecast



An informed estimate of the likely evolution of antisense oligonucleotide therapeutics, over the period of 2020-2030. The report provides likely distribution of the future opportunity across multiple market segments³

Oligonucleotide CMOs: Case Study



A brief case study on the oligonucleotide manufactures, including information on the year of establishment, company size, geographical location and method of purification used

Expert Opinions



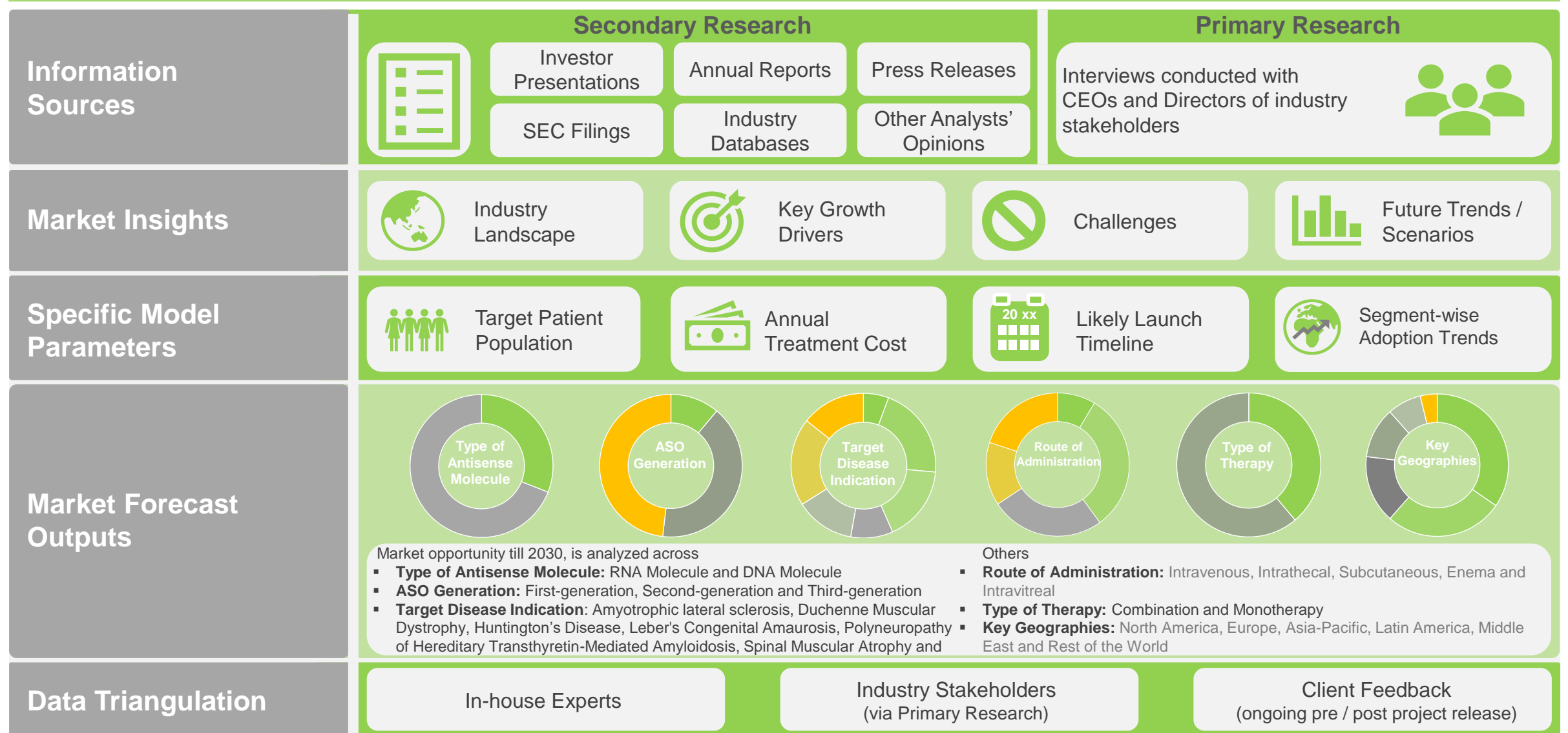
Views (sourced from videos, blogs and other public forums) of experts and key opinion leaders to understand their understanding of the industry and validate specific aspects of the report

Note 1: The parameters include information on various therapy developer companies, type of antisense molecule, ASO Generation, phase of development, target genes, target indications, therapeutic areas, route of administration, year of establishment, company size, and location of headquarters

Note 2: The parameters for grant analysis of antisense oligonucleotide therapeutics include year of award, amount awarded, administering institute centre, support period, type of grant application, purpose of grant, type of activity code, type of recipient organization and geographical distribution of the recipient organizations.

Note 3: The projected opportunity within antisense oligonucleotide therapeutics market has been analyzed across the following segments [A] Type of antisense molecule [B] ASO Generation [C] Target Disease Indication [D] Route of Administration [E] Type of Therapy [F] Key Geographies

Project Approach



Example highlights

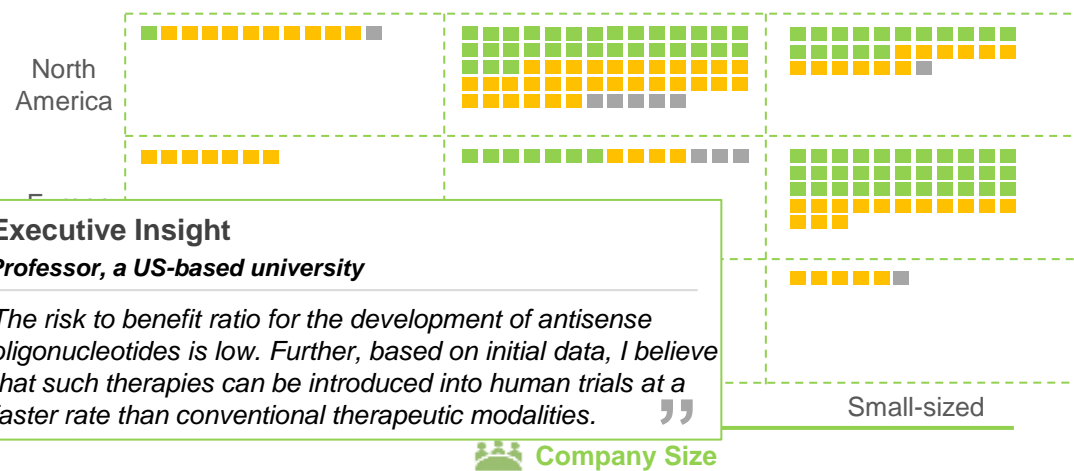
Around 30 players from across the world are presently engaged in evaluating the potential therapeutic benefits of antisense oligonucleotides for the treatment of a wide range of disease indications

List of Antisense Oligonucleotide Therapeutics

| S. No. | Therapy Name | Developer Specific Details | | | Therapy Specific Details | | | | | | |
|--------|---------------|----------------------------|------|----|--------------------------|--------------|-------------------------------------|----------------------------|-----------------|-------------|------------|
| | | Company | YoE | HQ | PoD | RoA | Indication | Type of Antisense Molecule | Type of Therapy | Target Gene | Generation |
| 1 | Alicaforsen | Atlantic Healthcare | 2006 | | Phase III | Intravenous | Crohn's disease | DNA molecule | Monotherapy | ICAM-1 | First |
| 4 | Danvatirsen | AstraZeneca | 1999 | | Phase II | Intravenous | Cancer | DNA molecule | Combination | STAT3 | Second |
| 7 | ISTH0036 | Company 7 | 1998 | | Phase I | Intravitreal | Glaucoma | DNA molecule | Monotherapy | TGF-β2 | Third |
| 13 | Tegsedi® | Company 13 | 1989 | | Marketed | Subcutaneous | Polyneuropathy of hATTR amyloidosis | RNA molecule | Monotherapy | TTR | Second |
| 20 | Prexigebersen | Bio-Path Holdings | 2007 | | Phase II | | | | | | |
| 32 | ION541 | Biogen | 1978 | | Phase I | | | | | | |
| 56 | GTX-102 | genetx | 2017 | | Phase I/II | | | | | | |
| 77 | Trabedersen | MATEON THERAPEUTICS | 1996 | | Preclinical | | | | | | |
| 94 | Sepaforsen | ProQR THERAPEUTICS | 2012 | | Phase II/III | | | | | | |
| 102 | ATL-1102 | Company 87 | 2000 | | Phase II | | | | | | |
| 115 | SRP-5052 | SAREPTA THERAPEUTICS | 1980 | | Preclinical | | | | | | |
| 123 | SB010 | stemo biologicals | 2006 | | Phase II | | | | | | |
| 156 | WVE-120102 | Company 127 | 2012 | | Phase I/II | | | | | | |
| 176 | QR-313 | WINGS BIOPHARMA | 2019 | | Phase I/II | | | | | | |

Distribution of Candidates by Phase of Development, Developer's Location and Company Size

Phase of Development | Preclinical / Research | Clinical | Marketed



Information on 170+ antisense oligonucleotide therapeutics is available in the detailed report

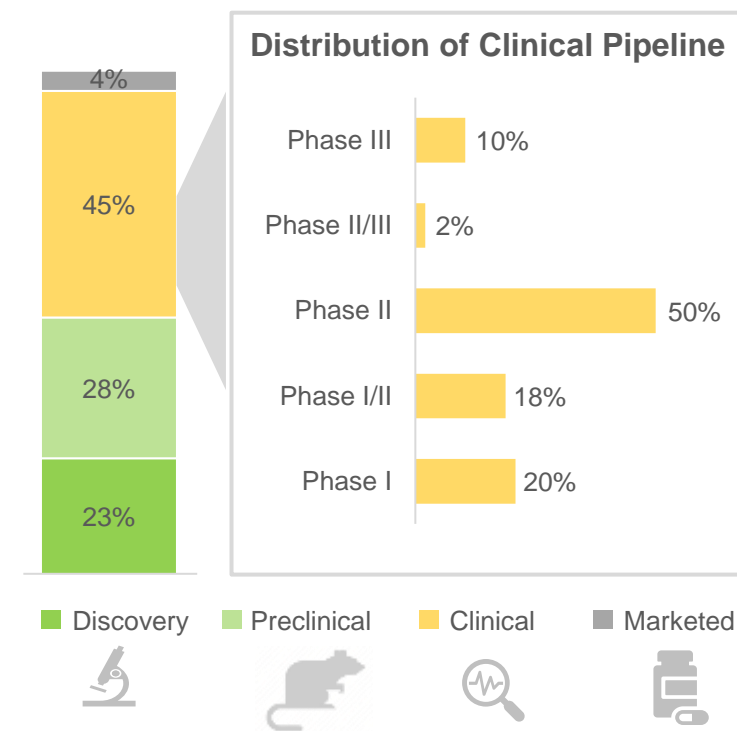
Abbreviations: YoE: Year of Establishment, HQ: Headquarters, PoD: Phase of Development, RoA: Route of Administration

Example highlights

The pipeline features 170+ candidate therapies in different stages of development, being evaluated either as mono-therapies or in combination with other interventions; most such products are administered parenterally

Antisense Oligonucleotide Therapeutics

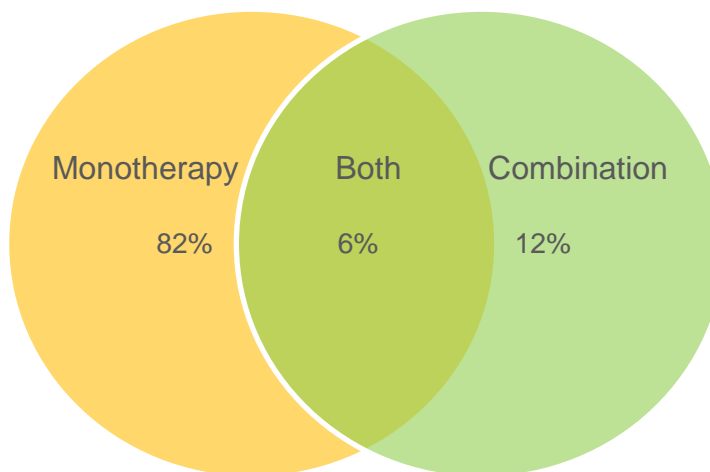
Distribution by Phase of Development



Majority of the approved therapies and late-stage candidates are intended for the treatment of genetic disorders, neurological disorders and oncological disorders

Antisense Oligonucleotide Therapeutics

Distribution by Type of Therapy



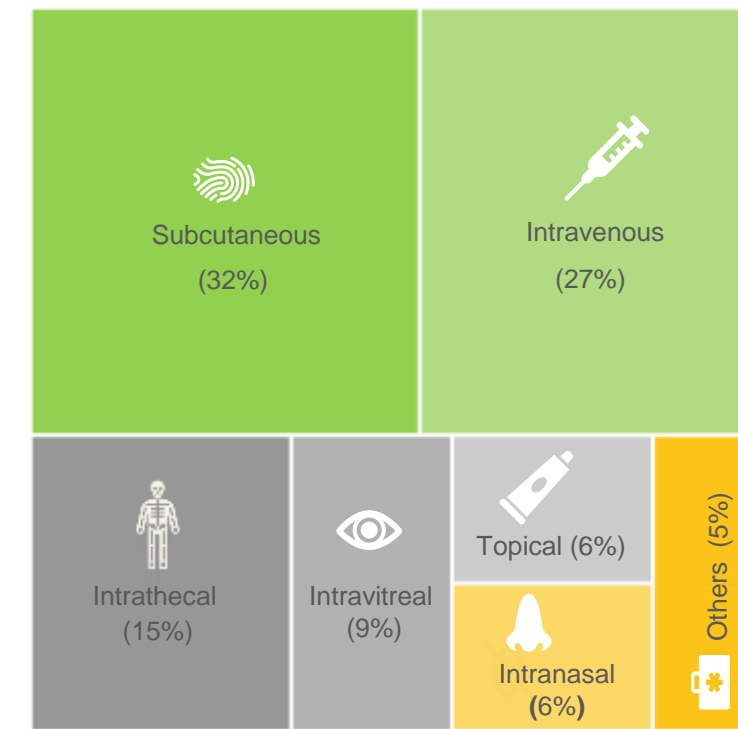
Examples of Candidates tested as Combination therapy

| Drug Name | Combination Therapy Name | Developer |
|---------------|--------------------------|-------------------|
| SPINRAZA® | ZOLGENSMA® | IONIS Biogen |
| Prexigebersen | DACOGEN® | Bio-Path Holdings |

Given the advantages of antisense oligonucleotides, these interventions are primarily evaluated as monotherapies. Late-stage drugs being investigated as monotherapy include Tofersen and Pelacarsen

Antisense Oligonucleotide Therapeutics

Distribution by Route of Administration¹



Majority of the antisense oligonucleotide therapeutics are designed for subcutaneous administration; these can be self-administered by the patients using different drug delivery systems

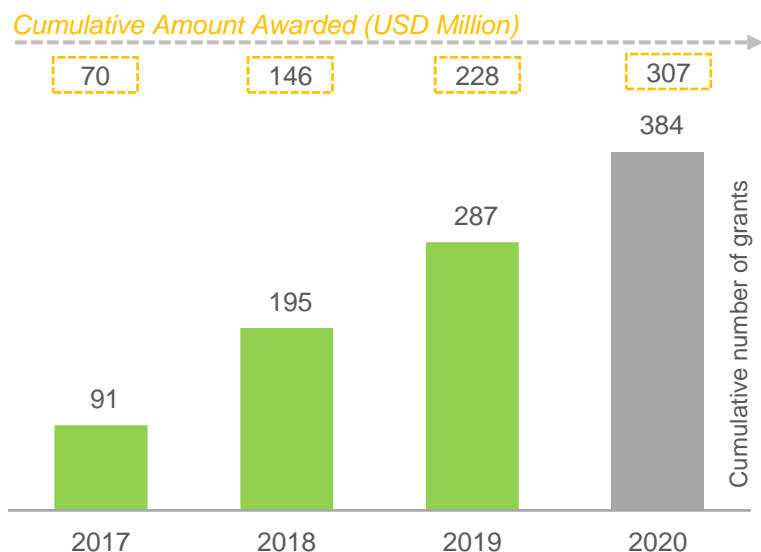
Note 1: The others category includes intratumoral, oral and intracerebroventricular routes

Example highlights

Several organizations have extended financial support to aid research efforts in this domain; currently, the focus, in terms of funds disbursed, is primarily in support of investigations of drugs for treating neurological conditions

Grant Analysis

Cumulative Year-wise Distribution



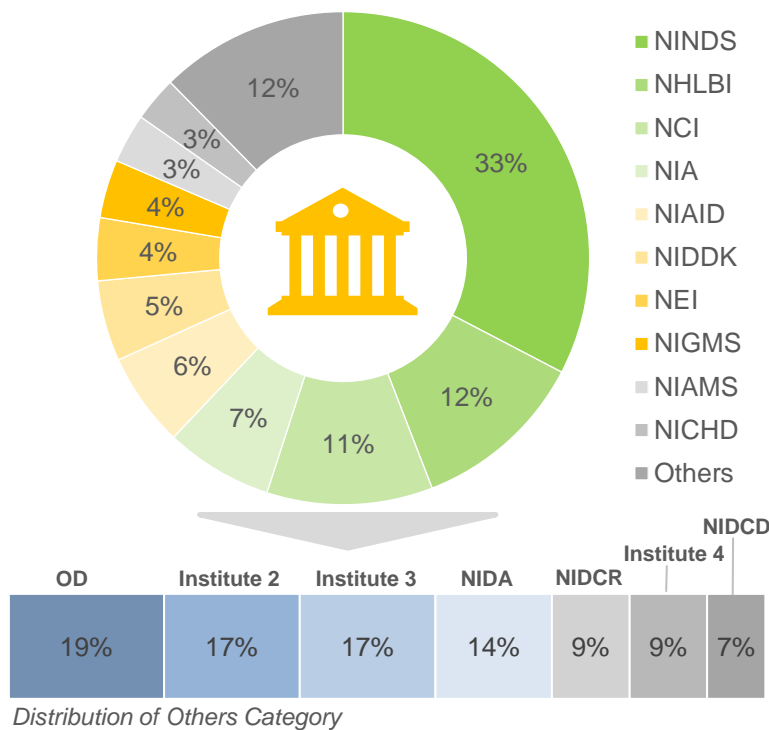
Executive Insight
 Chief Scientific Officer, a small-sized developer

“Several players across the globe are presently exploring the potential of antisense oligonucleotides to develop novel therapies against SARS-Cov2.”

The number of grants awarded to stakeholders in this domain (in the US) has continuously increased between 2017 and 2020; more than 70% of the total amount was awarded for research projects

Grant Analysis

Leading Funding Institute Centers



The field has witnessed the involvement of various administering institutes of the NIH; of all the institutes, participation of the NINDS, NHLBI, and NCI has been relatively more prominent

Grant Analysis

Word Cloud of Grant Titles



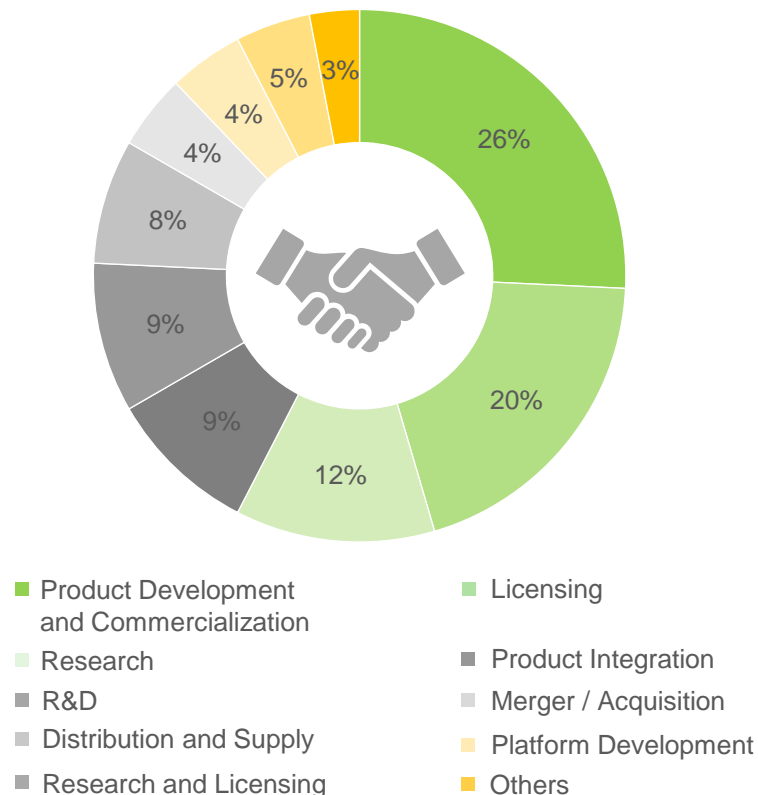
The word cloud represents the areas of interest of research organizations, within this emerging domain; multiple development initiatives are focused on therapeutic areas, such as SMA and dystrophy

Example highlights

The rising interest in this field is reflected in the number of partnerships inked by the various stakeholders across different application areas

Partnerships and Collaborations

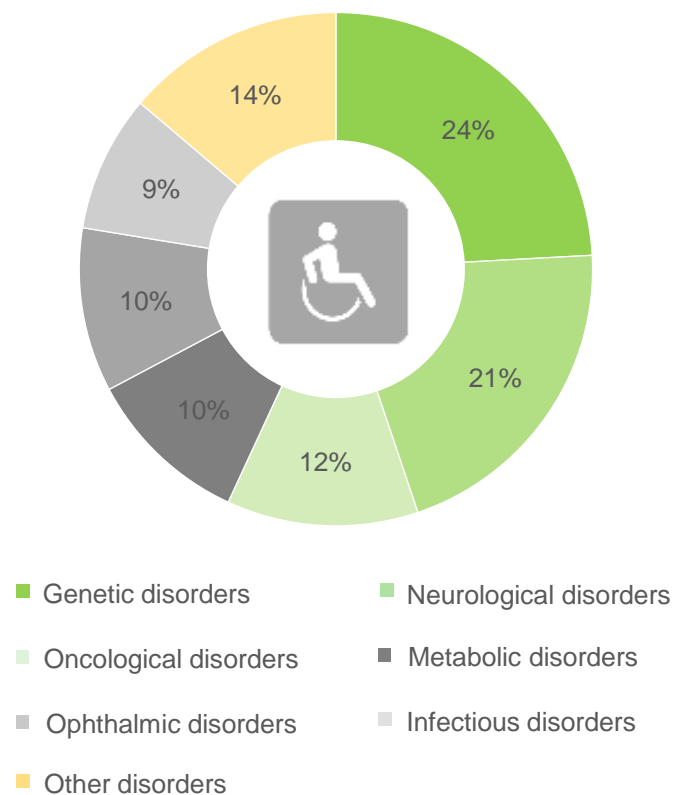
Distribution by Type of Partnership¹



Given that nearly 20 molecules are in the late stages of development, companies have mostly collaborated for product development and commercialisation

Partnerships and Collaborations

Distribution by Therapeutic Area¹



Both established players and the new entrants have forged strategic partnerships in the recent past; these deals have primarily been inked for genetic and neurological disorders

Partnerships and Collaborations

Recent Examples¹



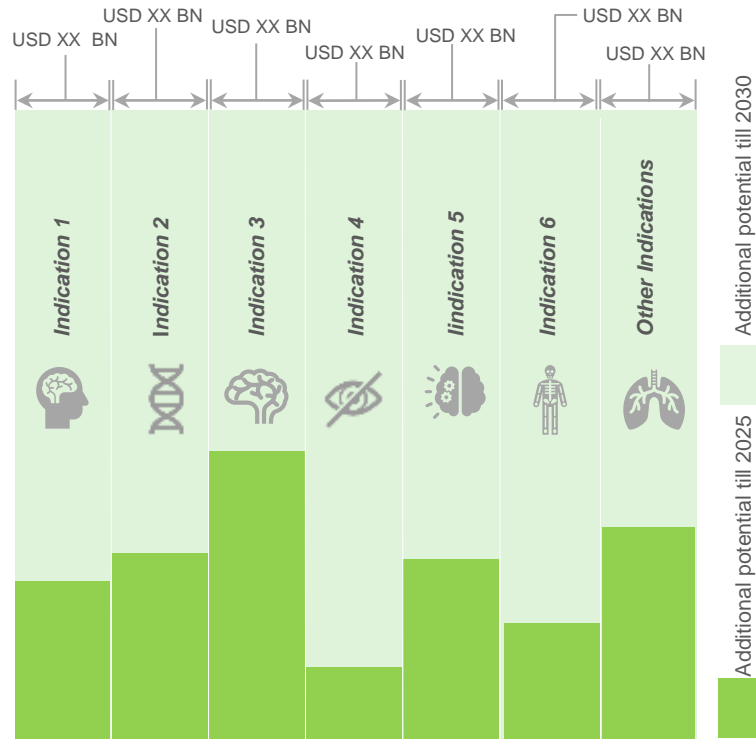
Note 1: Detailed analysis of partnerships (captured till October 2020) based on several relevant parameters, including type of partner, type of generation is available in the report titled “Antisense Oligonucleotide Therapeutics Market, 2021-2030”

Example highlights

The future opportunity, in terms of revenues from the sales of marketed and late-stage therapies, is anticipated to be well distributed across different disease areas, types of molecules and key geographical regions

Antisense Oligonucleotide Therapeutics Market

Distribution by Disease Indication^{1, 2}



Note 1: Illustrations are not as per actual scale

Note 2: The “Antisense Oligonucleotide Therapeutics Market, 2021-2030” report takes into consideration the following disease indications: Amyotrophic lateral sclerosis, Duchenne Muscular Dystrophy, Huntington’s Disease, Leber’s Congenital Amaurosis, Polyneuropathy of Hereditary Transthyretin-Mediated Amyloidosis and Spinal Muscular Atrophy. Other indications include Familial chylomicronemia syndrome, Familial Partial Lipodystrophy and Pouchitis

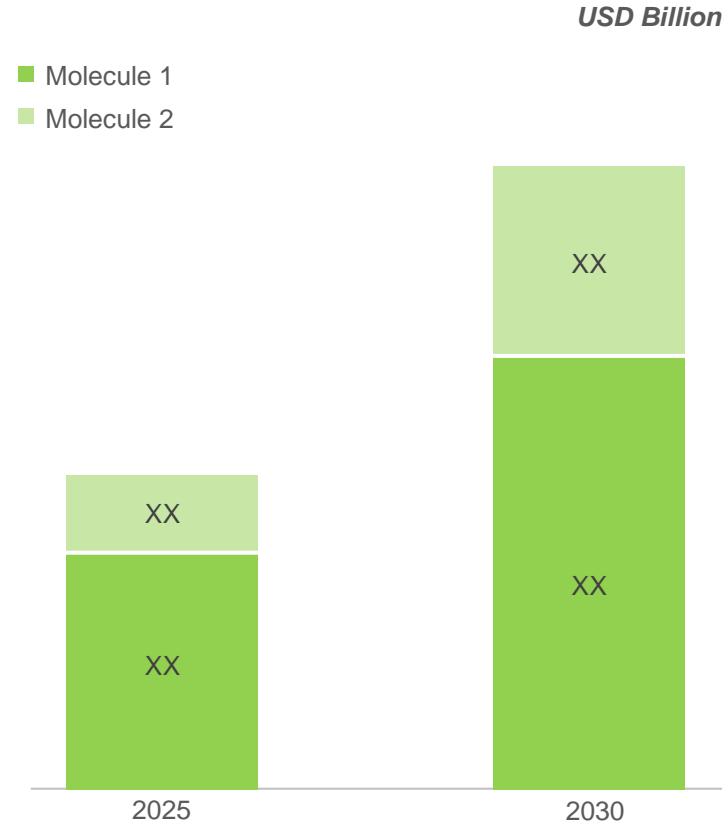
Note 3: The “Antisense Oligonucleotide Therapeutics Market, 2021-2030” report takes into consideration the following types of molecules: DNA molecule and RNA molecule

Note 4: The “Antisense Oligonucleotide Therapeutics Market, 2021-2030” report takes into consideration the following regions: North America, Europe, Asia-Pacific, Latin America, Middle East and North Africa, and Rest of the World

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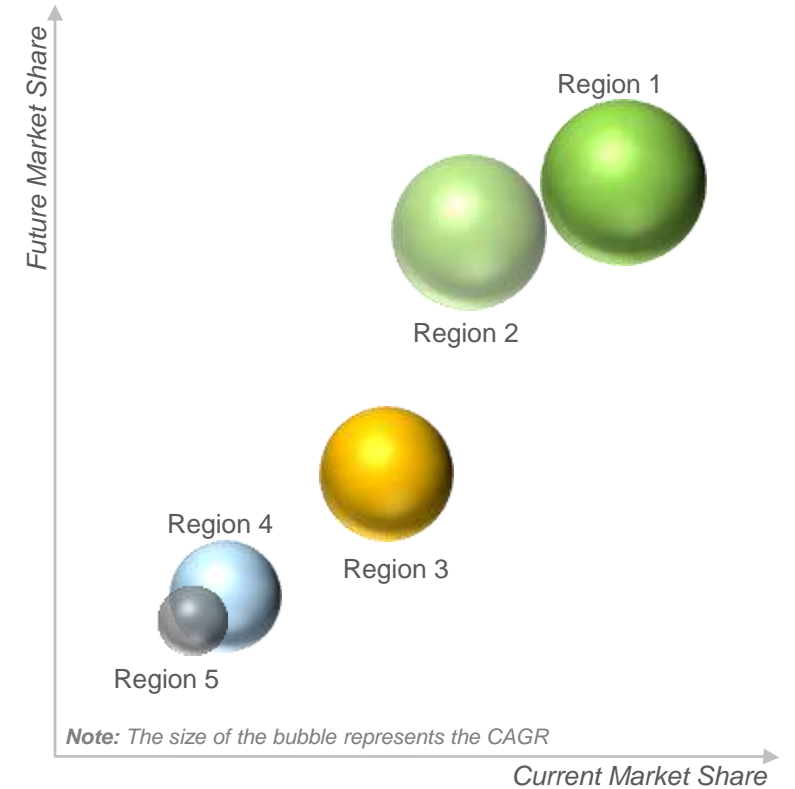
Antisense Oligonucleotide Therapeutics Market

Distribution by Type of Molecule^{1, 3}



Antisense Oligonucleotide Therapeutics Market

Distribution by Geography^{1, 4}



Note: The size of the bubble represents the CAGR

Example highlights

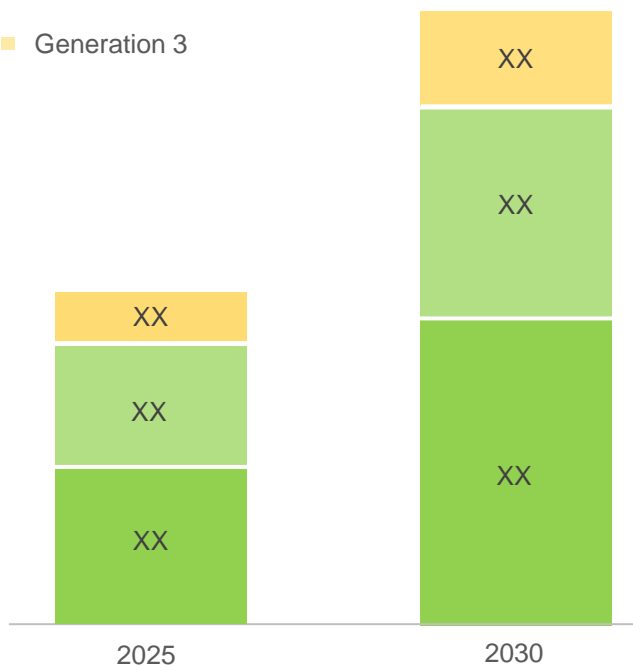
The market is likely to witness steady growth over the coming decade; the opportunity will be dispersed across different generations, routes of administration and various types of therapies

Antisense Oligonucleotide Therapeutics Market

Distribution by Generation^{1, 2}

USD Billion

- Generation 1
- Generation 2
- Generation 3

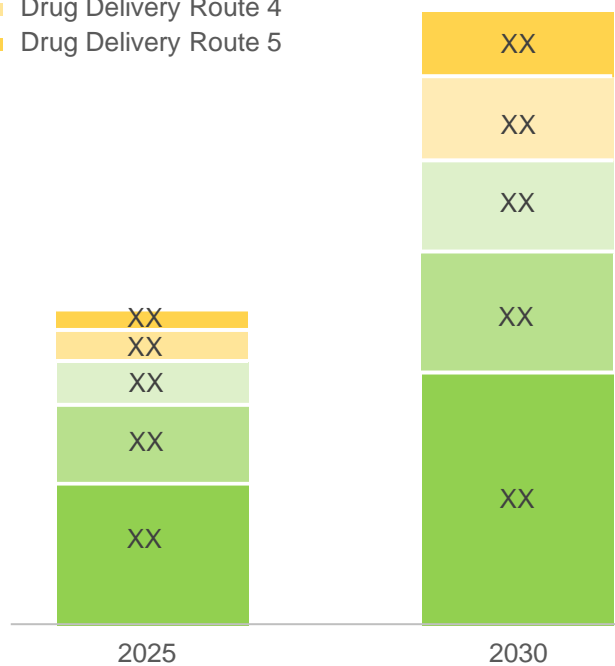


Antisense Oligonucleotide Therapeutics Market

Distribution by Drug Delivery Route^{1, 3}

USD Billion

- Drug Delivery Route 1
- Drug Delivery Route 2
- Drug Delivery Route 3
- Drug Delivery Route 4
- Drug Delivery Route 5

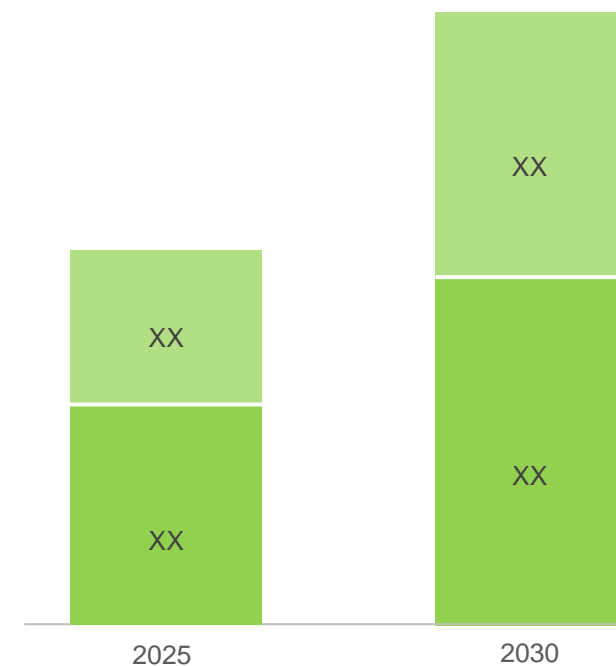


Antisense Oligonucleotide Therapeutics Market

Distribution by Type of Therapy^{1, 4}

USD Billion

- Therapy 1
- Therapy 2



Note 1: Illustrations are not as per actual scale

Note 2: The "Antisense Oligonucleotide Therapeutics Market, 2021-2030" takes into consideration the following generations of antisense molecules: First-generation, Second-generation and Third-generation

Note 3: The "Antisense Oligonucleotide Therapeutics Market, 2021-2030" takes into consideration the following routes of administration: Intravenous, Intrathecal, Subcutaneous, Enema and Intravitreal

Note 4: The "Antisense Oligonucleotide Therapeutics Market, 2021-2030" takes into consideration the following types of therapy: Monotherapy, and Combination therapy

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Chapter Outlines (1/1)

- **Chapter 2** provides an executive summary of the insights captured in our research. It presents a high-level view on the current scenario within the antisense oligonucleotide therapeutics market and its evolution in the short-mid-term and long term.
- **Chapter 3** presents a general introduction to oligonucleotides, laying emphasis on the antisense oligonucleotide therapeutics, including information on their mechanism of action and types. Additionally, the chapter features a brief discussion on the likely future trends in this field.
- **Chapter 4** provides information on more than 150 programs for antisense oligonucleotide therapeutics that are either approved or being developed across type of antisense molecule (RNA molecule and DNA molecule), ASO generation (first-generation, second-generation, third-generation and next-generation), phase of development (commercial, clinical, preclinical, and discovery stage) of lead candidates, target genes, target disease indications, target therapeutic areas, route of administration (subcutaneous, intravenous, intrathecal, intravitreal and others) and type of therapy (monotherapy, combination therapy and both). Additionally, the chapter includes information on drug developer(s), highlighting year of establishment, company size, and location of headquarters.
- **Chapter 5** provides tabulated profiles of the companies that are engaged in the development of at least two or more antisense oligonucleotide based therapies (in phase II and above). Each profile features a brief overview of the company, its financial information (if available), product portfolio, recent developments and an informed future outlook.
- **Chapter 6** provides a detailed analysis of completed, ongoing and planned clinical studies of various antisense oligonucleotide therapeutics, highlighting prevalent trends across various relevant parameters, such as trial registration year, phase of development, current trial status, enrolled patient population, study design, leading industry sponsors / collaborators (in terms of number of trials conducted), trial focus, target therapeutic area, target genes, popular indications, popular products and regional distribution of trials.
- **Chapter 7** provides an analysis of more than 380 grants that were awarded to research institutes engaged in antisense oligonucleotide therapeutics, in the period between 2017 and 2019 (till September) based on the important parameters, such year of grant award, amount awarded, administering institute center, support period, type of grant application, purpose of grant award, activity code, focus area, study section involved, and type of recipient organizations. In addition, it highlights popular target therapeutic areas, popular funding institute centers, prominent program officers, and popular recipient organizations.
- **Chapter 8** features a discussion of the various collaborations and partnerships that have been inked amongst stakeholders in this domain, since 2016. It includes a brief description of various types of partnership models (namely acquisitions and mergers, licensing agreements, product development agreements, research agreements, joint venture agreements and other agreements) that have been adopted by stakeholders in this domain. In addition, it includes a detailed analysis of partnerships, based on year of partnership, type of partnership, and regional activity.
- **Chapter 9** features a detailed market forecast analysis, highlighting the likely growth of antisense oligonucleotide therapeutics till the year 2030. The chapter presents a detailed market segmentation on the basis of type of antisense molecule (RNA and DNA molecule), different target indications (duchenne muscular dystrophy, spinal muscular atrophy, hereditary transthyretin-mediated (hATTR) amyloidosis, familial chylomicronemia syndrome, familial partial lipodystrophy, pouchitis, leber's congenital amaurosis, huntington's disease and amyotrophic lateral sclerosis), ASO generations (first-generation, second-generation and third-generation), route of administration (intrathecal, intravenous, intravitreal, subcutaneous, and topical), type of therapy (combination therapy and monotherapy) and key geographical regions (US, UK, EU4, Asia-Pacific and rest of the world) In order to account for future uncertainties and to add robustness to our model, we have provided three market forecast scenarios, namely conservative, base and optimistic scenarios, representing different tracks of the industry's growth.
- **Chapter 10** provides insights on current market landscape of oligonucleotide manufacturers focused on research and diagnostic, and therapeutic applications including information on the year of establishment, company size, scale of operation (small, medium, and large), location of headquarters and type of purification methods used.
- **Chapter 11** is a summary of the overall report, wherein we have mentioned all the key facts and figures described in the previous chapters. The chapter also highlights the evolutionary trends that were identified during the course of the study and are expected to influence the future of the antisense oligonucleotide therapeutics market.
- **Chapter 12** is an appendix, which provides tabulated data and numbers for all the figures included in the report.

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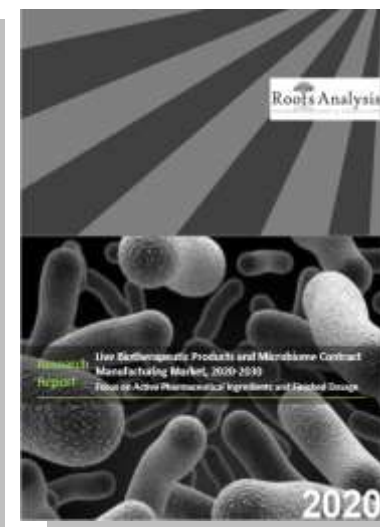
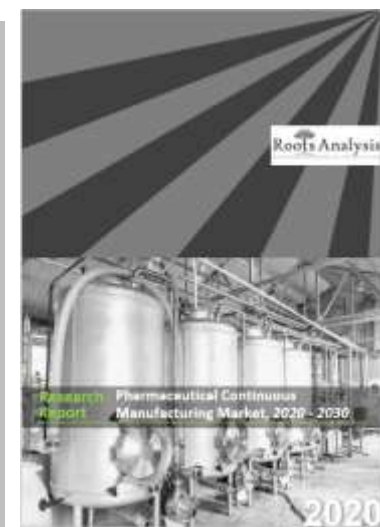
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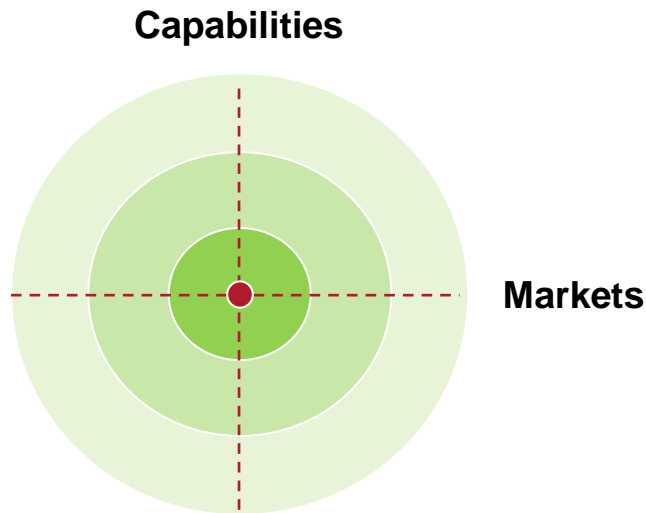
1. Abbott
2. Acedra BioPharmaceutical
3. ACGT
4. Achieve Life Sciences
5. Agilent Technologies
6. Ajinomoto Bio-Pharma Services
7. Akcea Therapeutics
8. Alcyone Lifesciences
9. A Biotechnology & Pharmaceuticals
10. Allianz BioInnovation
11. Alta Bioscience
12. AM Chemicals
13. AmpTec
14. Amylon Therapeutics
15. Antisense Therapeutics
16. Applied Biological Materials
17. Aro Biotherapeutics
18. AstraZeneca
19. ATDBio
20. Atlantic Healthcare
21. ATZ Labs
22. Axolabs
23. Bachem
24. Bayer
25. BiancoScience
26. Bio Basic
27. Biogen
28. Biologio
29. BioMarin Pharmaceutical
30. Bioneer
31. Bio-Path Holdings
32. Biosearch Technologies
33. BioServe Biotechnologies
34. 2BioSpring
35. Bio-Synthesis
36. Boston Children's Hospital
37. BR Biochem Life Sciences
38. Bridgen
39. Catabasis Pharmaceuticals
40. ChemGenes
41. Codiak BioSciences
42. Cold Spring Harbor Laboratory
43. CordenPharma
44. Creative Biogene
45. CUSABIO TECHNOLOGY
46. CyberGene
47. Daiichi Sankyo
48. Dalton Pharma Services
49. Deep Genomics
50. Denali Therapeutics
51. Dharmacon
52. Duke University
53. Dynacure
54. EB Research Partnership
55. Eli Lilly and Company
56. Elim Biopharmaceuticals
57. Empirico
58. Eton Bioscience
59. Eurofins Genomics
60. Evotec
61. Evox Therapeutics
62. F. Hoffmn-La-Roche
63. Firebrand Therapeutics
64. F-Star Therapeutics
65. FUTUREsynthesis
66. GCC Biotech
67. Genbiotech
68. Gene Signal
69. Gene Universal
70. GeneDesign
71. Genei Laboratories
72. GenePharma
73. General Biosystems
74. GENERI BIOTECH
75. GeneTx Biotherapeutics
76. GENEWIZ
77. GenScript
78. Genuity Science
79. GlaxoSmithKline
80. Golden Mountain Partners
81. Hanugen Therapeutics
82. IBA Lifesciences
83. inqaba biotec™
84. Integrated DNA Technologies
85. Ionis Pharmaceuticals
86. Isarna Therapeutics
87. Janssen Biotech
88. Johns Hopkins University
89. Johnson Matthey
90. Kaneka Eurogentec
91. KareBay Biochem
92. Lions Eye Institute
93. Lipigon Pharmaceuticals
94. Lonza
95. Mateon Therapeutics
96. Medical Need
97. Medigene
98. Microsynth
99. Midland Certified Reagent
100. Moligo Technologies
101. National Cancer Institute
102. National Institute of Allergy and Infectious Diseases
103. NeuBase Therapeutics
104. Nippon Shinyaku
105. Nitto Denko Avecia
106. Novartis
107. Novatia
108. NZYTech
109. Oligomer
110. OncoGenex Pharmaceuticals
111. Oncolteic
112. Oregon Health & Science University
113. Parexel
114. Pfizer
115. Primetech
116. Prometheus Research
117. ProQR Therapeutics
118. PTC Therapeutics
119. PureTech Health
120. PYC Therapeutics
121. Quintara Biosciences
122. ReadCoor
123. Rena Therapeutics
124. Rexahn Pharmaceuticals
125. Rockland Immunochemicals
126. RogCon Biosciences
127. RuiBo Bio-Technology
128. Sarepta Therapeutics
129. Scandinavian Gene Synthesis
130. Secarna Pharmaceuticals
131. Severn Biotech
132. Sigma Aldrich
133. Spring Bank Pharmaceuticals
134. SRI International
135. ST Pharm
136. STA Pharmaceuticals
137. Stanford University
138. Sterna Biologicals
139. Stoke Therapeutics
140. Sumitomo Chemical
141. Sun Yat-sen University
142. Sunomix Biosciences
143. Suzhou Ribo Life Science
144. Synbio Technologies
145. Syngene International
146. TAG Copenhagen
147. Takeda Pharmaceutical
148. The University of Texas MD Anderson Cancer Center
149. The University of Texas Southwestern Medical Center
150. Thermo Fisher Scientific
151. Thomas Jefferson University
152. TriLink Biotechnologies
153. Ultragenyx Pharmaceutical
154. United States Army Medical Research Institute of Infectious Diseases
155. United States Department of Defense
156. University of California
157. University of Florida
158. University of Massachusetts Medical School
159. University of Rochester
160. Vallentech
161. Vivantis Technologies
162. Wave Life Sciences
163. Wings Therapeutics
164. Xcelris
165. Zaphyr Pharmaceuticals
166. Zhejiang Haichang Biotechnology

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We provide deep expertise, experience and knowledge in bio-pharmaceutical and allied markets



Example Markets

- Diagnostics
- Emerging technologies
- Medical devices
- Therapeutic areas
- Drug delivery
- Clinical trials
- Health & wellness
- Pharmaceutical packaging

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- Market landscaping
- Competitive analysis
- 'Value chain' analysis
- Financial and business planning
- Technology / product benchmarking
- Drug pipeline evaluation
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