



Gene, Cell, & RNA Therapy Landscape

Q1 2021 Quarterly Data Report

Q1 2021





About the authors

The American Society of Gene & Cell Therapy (ASGCT) is the primary professional membership organization for scientists, physicians, patient advocates, and other professionals with interest in gene and cell therapy.

Our members work in a wide range of settings including universities, hospitals, government agencies, foundations, biotechnology and pharmaceutical companies. ASGCT advances knowledge, awareness, and education leading to the discovery and clinical application of gene and cell therapies to alleviate human disease to benefit patients and society. For more information visit [ASGCT.org](https://www.ASGCT.org).



Informa Pharma Intelligence powers a full suite of analysis products – Datamonitor Healthcare™, Sitetrove™, Trialtrove™, Pharmaprojects™, Biomedtracker™, Scrip™, Pink Sheet™ and In Vivo™ – to deliver the data needed by the pharmaceutical and biomedical industry to make decisions and create real-world opportunities for growth.

With more than 400 analysts, journalists, and consultants keeping their fingers on the pulse of the industry, no key disease, clinical trial, drug approval or R&D project isn't covered through the breadth and depth of data available to customers. For more information visit [pharmaintelligence.informa.com](https://www.pharmaintelligence.informa.com).

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A microscopic view of numerous cells, likely red blood cells, showing their characteristic biconcave disc shape. The cells are densely packed and appear to be in a fluid medium, with a warm, reddish-orange color palette. The lighting highlights the texture of the cell membranes and the darker centers.

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Welcome from ASGCT

Welcome to the first in a series of quarterly reports on the clinical development of the gene therapy field. ASGCT is thrilled to partner with Informa Pharma Intelligence to provide a data-driven, comprehensive look at clinical and pre-clinical progress across gene and cell therapies throughout the world to our members, and to the community at large.

This collection of reports will grow over time to show the expansion of the field, reflect clinical progress, and reveal patterns in therapeutic areas under development.

To that end, the data show unequivocally that gene, cell, and RNA therapies continue to grow rapidly with nearly 3,500 therapies in preclinical and clinical development throughout the world. While the overwhelming majority of therapies target cancers (and the second most common therapeutic target is neurological disorders), there are currently 77 therapies in Phase III clinical trials across all gene, cell, and RNA therapies.

Perhaps most impressive in all this report, nearly \$480M in start-up financing has been acquired across 13 companies throughout the gene, cell, and RNA therapeutics industry.

ASGCT extends a massive thanks to our data partners at Informa Pharma Intelligence in collecting and curating all this revealing information, and together we look forward to your response to this and future reports.

Key takeaways from Q1 2021

Oncology remains most active therapeutic area

- More than 1,200 therapies are in development targeting cancer at the end of Q1 2021 (preclinical to preregistration)

Neurological disease (unspecified) is the most common non-oncology disease area targeted by gene therapy

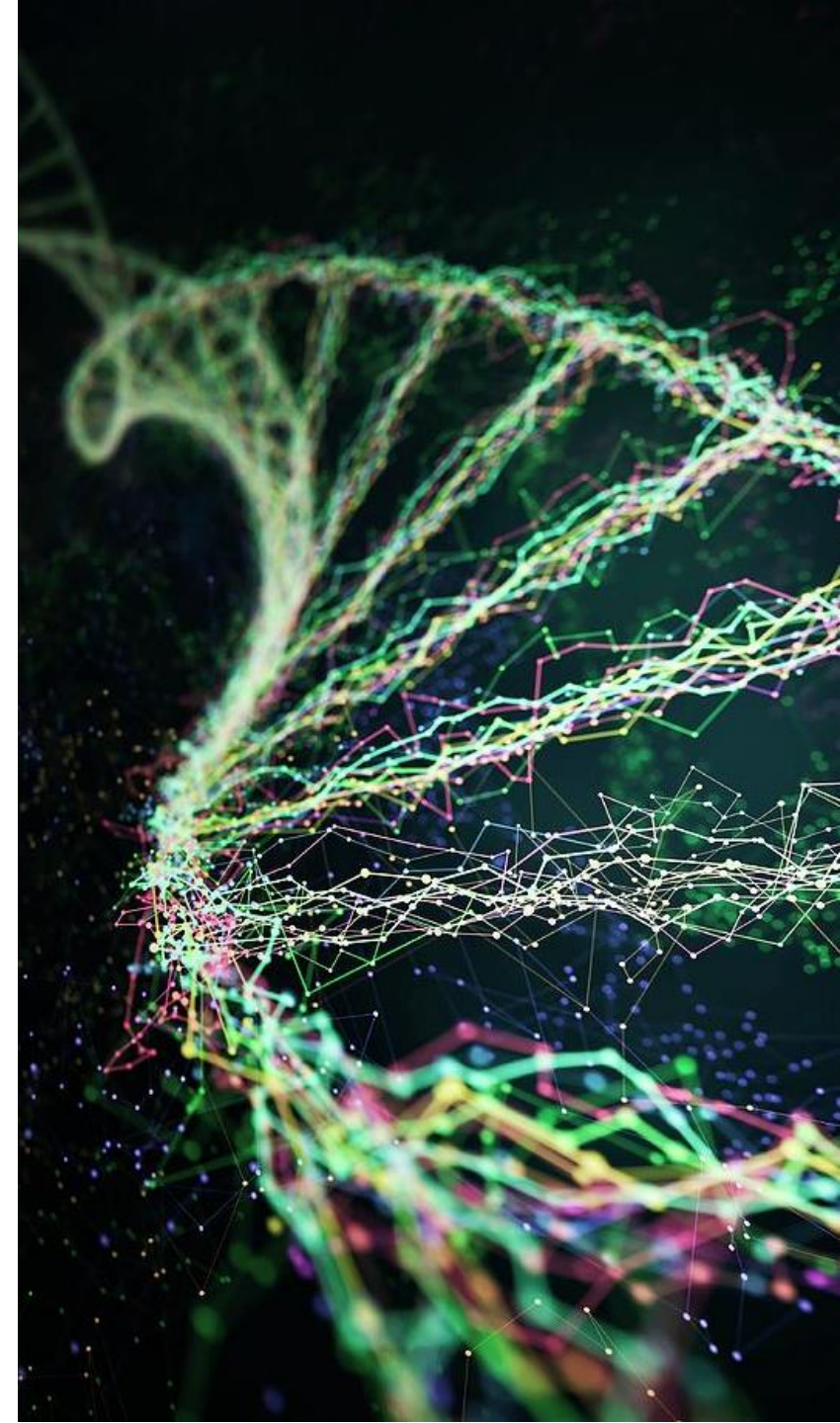
- 35 therapies are in development (preclinical to preregistration stage)

Astellas is most active preclinical developer

- Firm has 23 candidates currently in preclinical development
- Next most active companies: Sarepta, China Immunotech, Taysha Gene Therapies, Yake, Bayer, and Takeda

The volume of start-up financings for gene, cell, and RNA therapy companies—13 companies—decreased from Q4 2020 but increased from a year ago

- Gene, cell, and RNA therapy start-up companies raised an aggregate \$480M through seed and Series A financings, including a pair of \$85M Series A rounds from Notch Therapeutics and LEXEO Therapeutics



Approved therapies

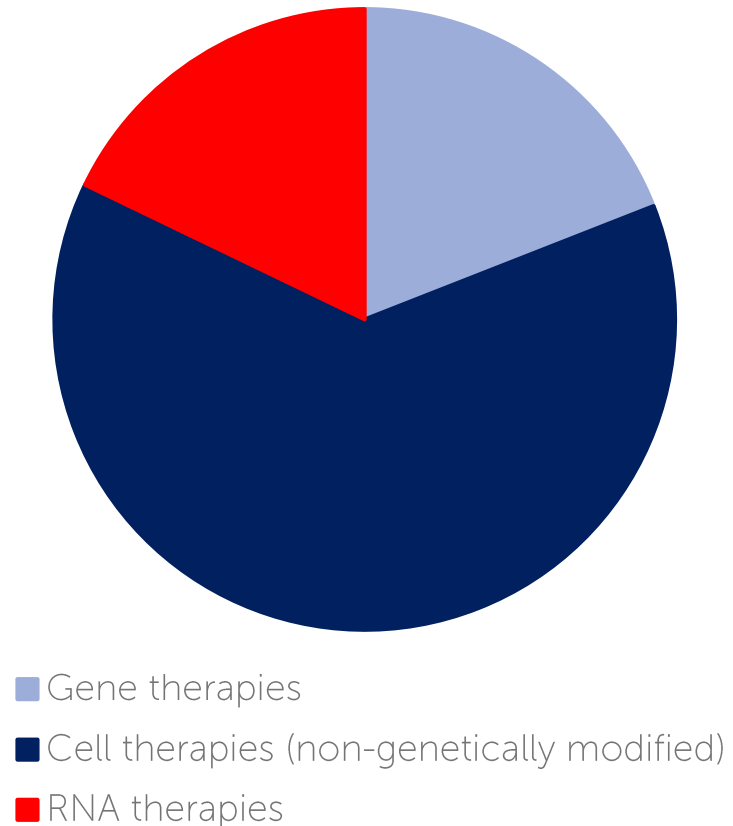
Q1 2021

Approved gene, cell, and RNA therapies

Globally, for clinical use, there are:

- 16 gene therapies approved (including genetically modified cell therapies)
- 15 RNA therapies approved
- 53 non-genetically modified cell therapies approved

Approved therapies by category



Source: Pharmaprojects | Informa, April 2021

Approved gene therapies

| Product name | Generic name | Year first approved | Disease(s) | Countries approved | Originator company |
|--------------|---|---------------------|--|--------------------|--------------------------------|
| Gendicine | recombinant p53 gene | 2004 | Head and neck cancer | China | Shenzhen SiBiono GeneTech |
| Oncorine | E1B/E3 deficient adenovirus | 2005 | Head and neck cancer; nasopharyngeal cancer | China | Shanghai Sunway Biotech |
| Rexin-G | mutant cyclin-G1 gene | 2006 | Solid tumors | Philippines | Epeius Biotechnologies |
| Neovasculgen | vascular endothelial growth factor gene | 2011 | Peripheral vascular disease; limb ischemia | Russian Federation | Human Stem Cells Institute |
| Imlygic | talimogene laherparepvec | 2015 | Melanoma | US, EU | Amgen |
| Strimvelis | autologous CD34+ enriched cells | 2016 | Adenosine deaminase deficiency | EU | Orchard Therapeutics |
| Kymriah | tisagenlecleucel-t | 2017 | Acute lymphoblastic leukemia; diffuse large B-cell lymphoma, including follicular lymphoma | US, EU, Japan | Novartis |
| Luxturna | voretigene neparvovec | 2017 | Leber's congenital amaurosis; retinitis pigmentosa | US, EU | Spark Therapeutics (Roche) |
| Yescarta | axicabtagene ciloleucel | 2017 | Diffuse large B-cell lymphoma, including DLBCL arising from follicular lymphoma. | US, EU | Kite Pharma (Gilead) |
| Collategene | bepermiogene perplasmid | 2019 | Critical limb ischemia | Japan | AnGes |
| Zolgensma | onasemnogene abeparvovec | 2019 | Spinal muscular atrophy | US, EU, Japan | Novartis |
| Zynteglo | lentiviral beta-globin gene transfer | 2019 | Transfusion-dependent beta thalassemia | EU | bluebird bio |
| Tecartus | brexucabtagene autoleucel | 2020 | Mantle cell lymphoma | US | Kite Pharma (Gilead) |
| Libmeldy | OTL-200 | 2020 | Metachromatic leukodystrophy | EU | Orchard Therapeutics |
| Breyanzi | lisocabtagene maraleucel | 2021 | DLBCL, including DLBCL arising from indolent lymphoma and follicular lymphoma grade 3B | US | Celgene (Bristol Myers Squibb) |
| Abecma | idecabtagene vicleucel | 2021 | Multiple myeloma | US | bluebird bio |

Source: [Pharmaprojects](#), [Biomedtracker](#) | Informa, April 2021

Approved RNA therapies

| Product name | Generic name | Year first approved | Disease(s) | Countries approved | Originator company |
|--------------------------|----------------------------|---------------------|--|---|-----------------------|
| Kynamro | mipomersen sodium | 2013 | Homozygous familial hypercholesterolaemia | US, Argentina, South Korea | Ionis Pharmaceuticals |
| Exondys 51 | eteplirsen | 2016 | Dystrophy, Duchenne muscular | US | Sarepta Therapeutics |
| Spinraza | nusinersen | 2016 | Muscular atrophy, spinal | US, Australia, Canada, EU, Japan, UK | Ionis Pharmaceuticals |
| Tegsedi | inotersen | 2018 | Amyloidosis, transthyretin-related hereditary | US, Canada, EU, UK | Ionis Pharmaceuticals |
| Onpattro | patisiran | 2018 | Amyloidosis, transthyretin-related hereditary | US, Canada, EU, Japan, UK | Alnylam |
| Vyondys 53 | golodirsen | 2019 | Dystrophy, Duchenne muscular | US | Sarepta Therapeutics |
| Waylivra | volanesorsen | 2019 | Hypertriglyceridaemia; Lipoprotein lipase deficiency | EU, UK | Ionis Pharmaceuticals |
| Comirnaty | COVID-19 vaccine, BioNTech | 2020 | Infection, coronavirus, novel coronavirus prophylaxis | US, Canada, EU, Iceland, Japan, Mexico, New Zealand, Rwanda, Singapore, UAE, UK | BioNTech |
| Moderna COVID-19 vaccine | COVID-19 vaccine, Moderna | 2020 | Infection, coronavirus, novel coronavirus prophylaxis | US, Canada, Hungary, Israel | Moderna Therapeutics |
| Givlaari | givosiran | 2020 | Porphyria | US, Germany, Italy | Alnylam |
| Oxlumo | lumasiran | 2020 | Hyperoxaluria | US, Germany | Alnylam |
| Ampligen | rintatolimod | 2020 | Chronic fatigue syndrome | Argentina | AIM ImmunoTech |
| Viltepso | viltolarsen | 2020 | Dystrophy, Duchenne muscular | US, Japan | NS Pharma |
| Leqvio | inclisiran | 2020 | Atherosclerosis; Heterozygous familial hypercholesterolaemia; Hypercholesterolaemia; Homozygous familial hypercholesterolaemia | EU, UK | Alnylam |
| Amondys 45 | casimersen | 2021 | Dystrophy, Duchenne muscular | US | Sarepta Therapeutics |

Source: [Pharmaprojects](#), [Biomedtracker](#) | Informa, April 2021

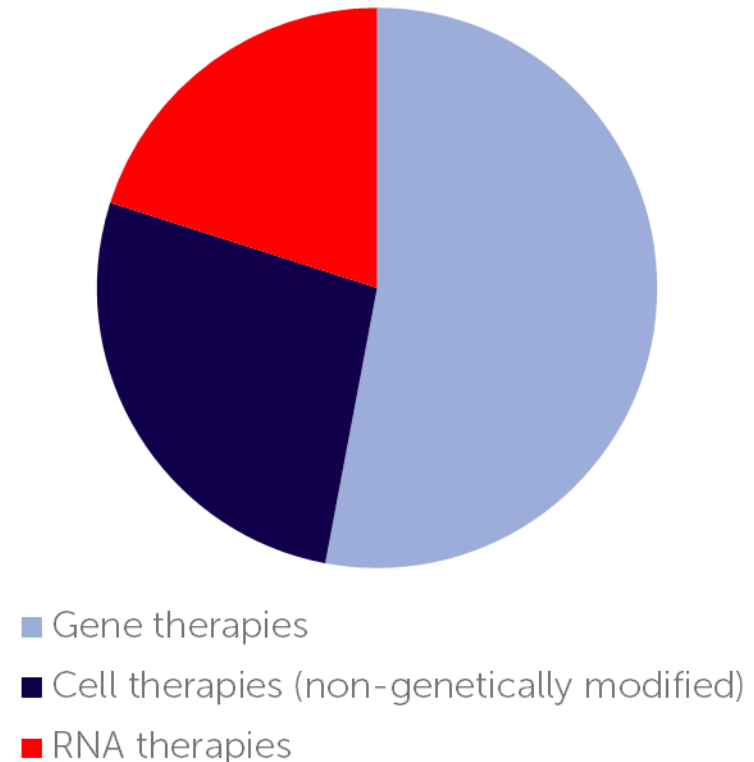
Pipeline overview

Q1 2021

Pipeline of gene, cell, and RNA therapies

- 3,474 therapies in development, ranging from preclinical through preregistration
- Gene therapy, including genetically-modified cell therapies such as CAR T-cell therapies, account for 53% of therapies in development
- Non-genetically modified cell therapies account for 27% of gene, cell, and RNA therapies

Pipeline therapies by category



Source: Pharmaprojects| Informa, April 2021

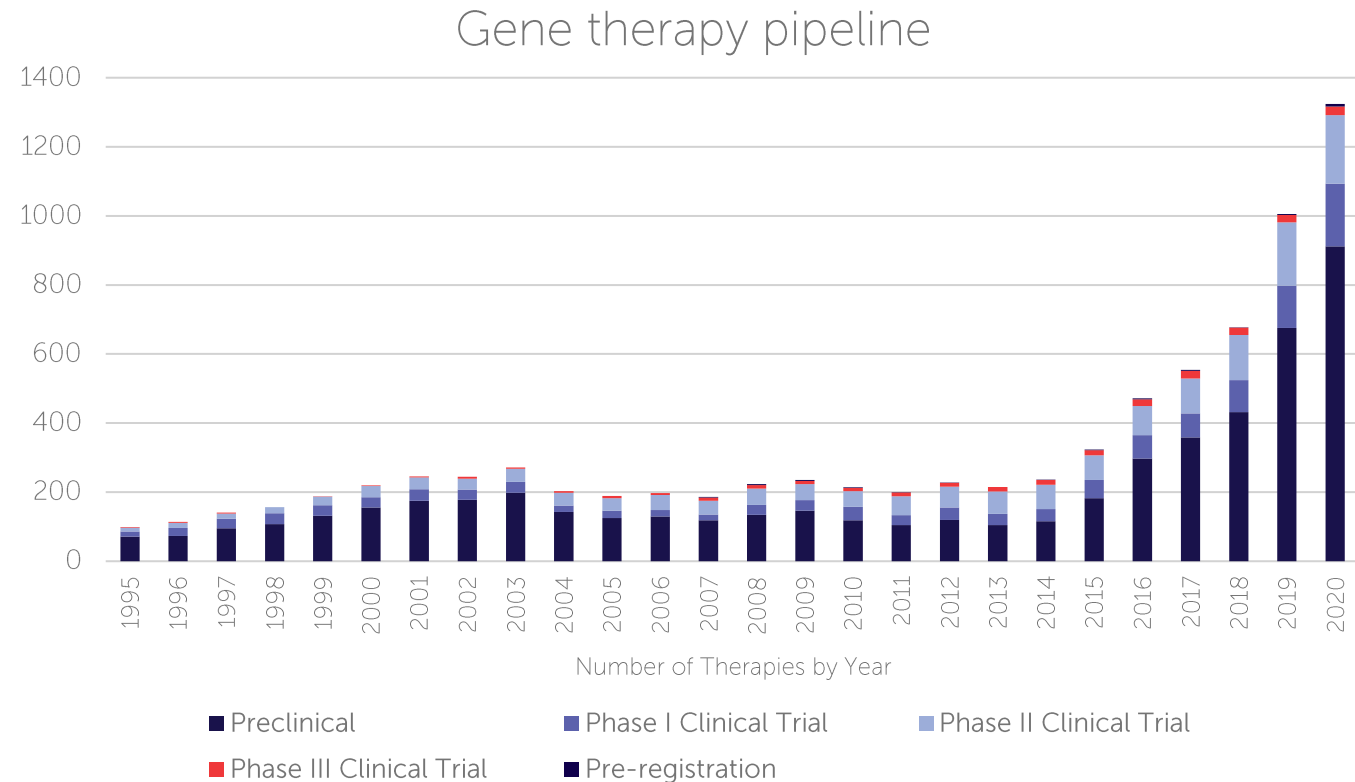
Gene therapy pipeline

Gene therapy and genetically modified cell therapies

Q1 2021

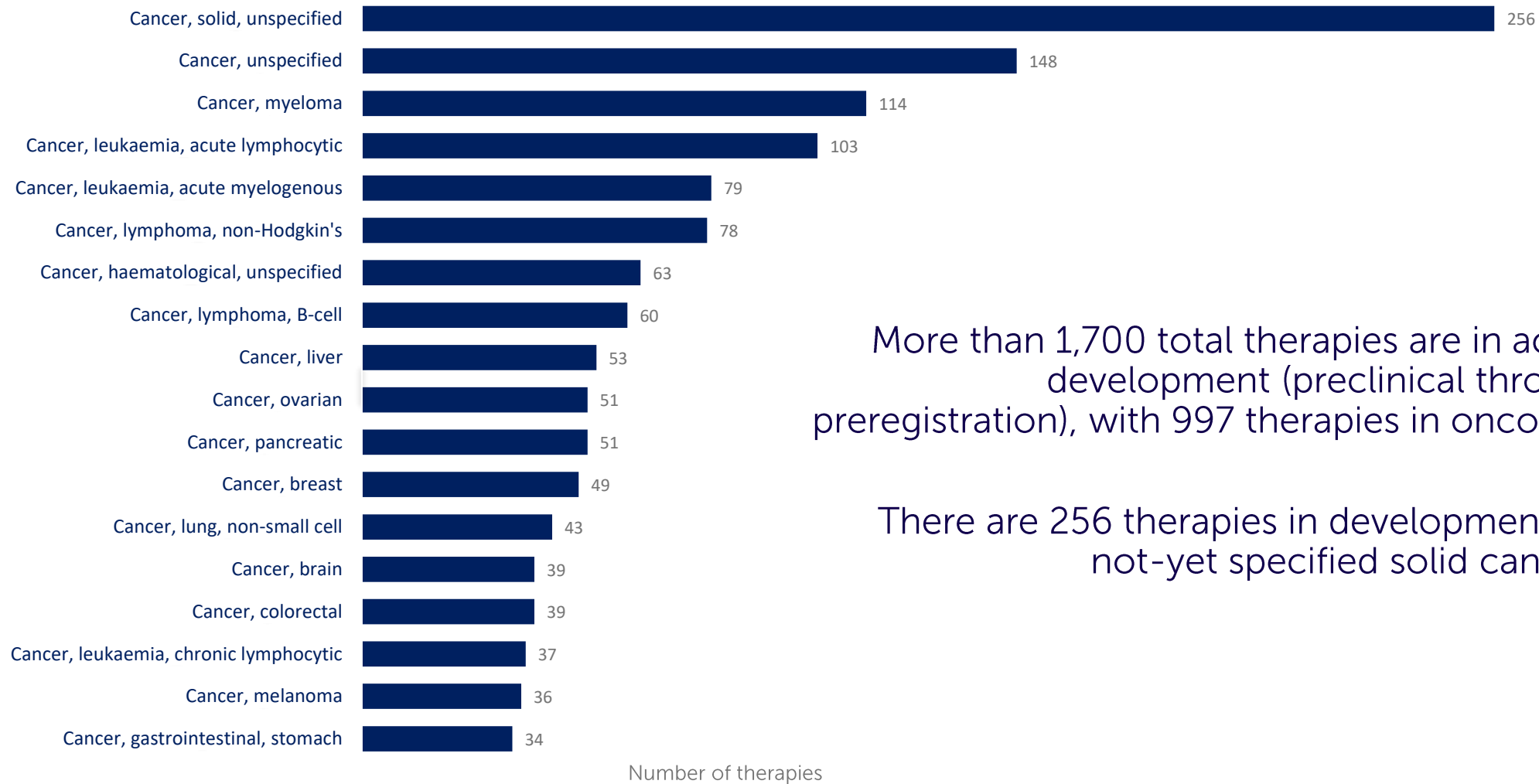
Gene therapy pipeline

- Number of gene therapies, including genetically modified cell therapies, has grown exponentially since 2014
- Preclinical pipeline supports early-stage discoveries
- In 2020:
 - Of more than 1,300 therapies in development (preclinical through preregistration), 69% of candidates were in preclinical development
 - 25 therapies were in Phase III clinical development



Source: Pharmaprojects| Informa, April 2021

Gene therapy pipeline: Most common cancers targeted

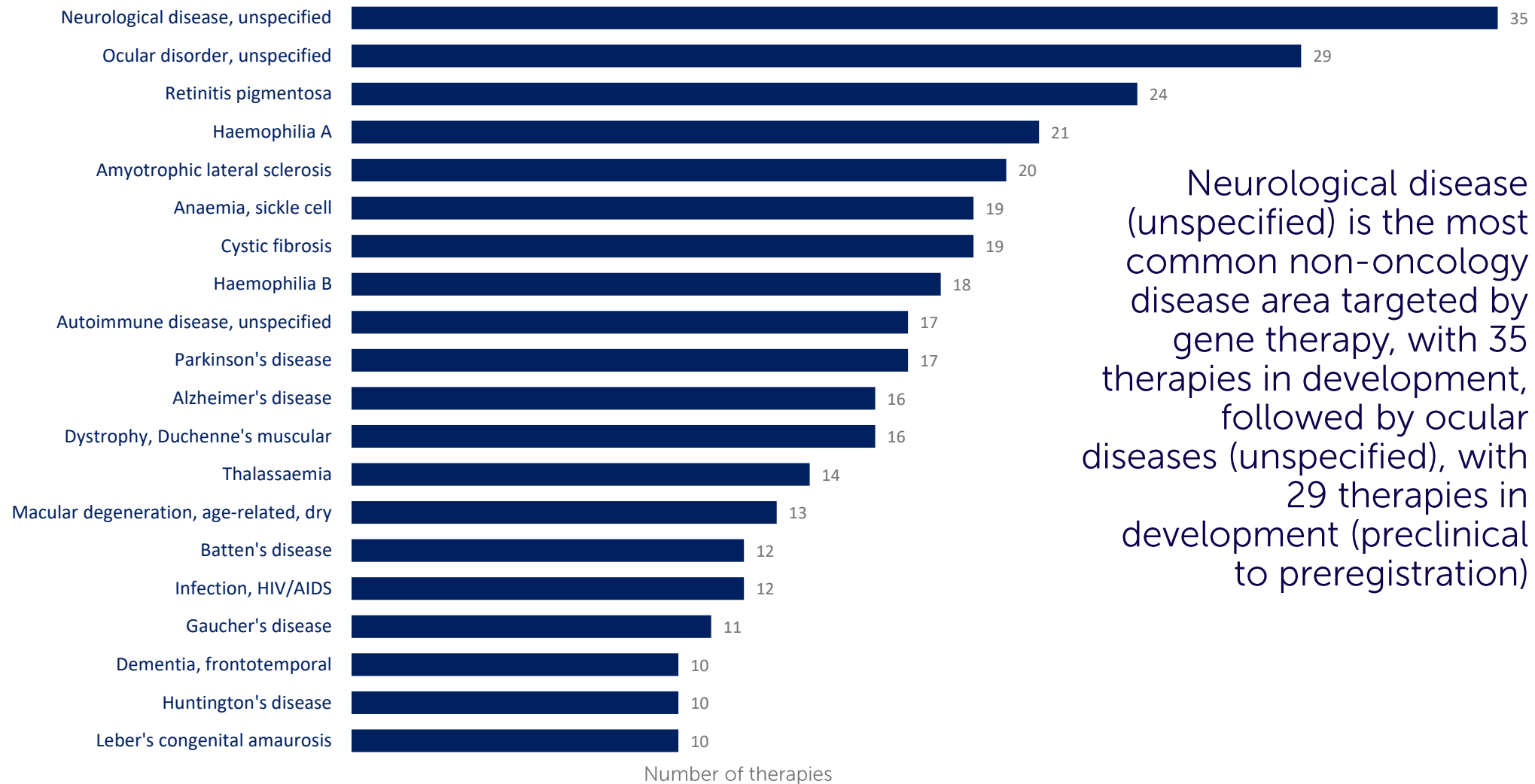


More than 1,700 total therapies are in active development (preclinical through preregistration), with 997 therapies in oncology

There are 256 therapies in development for not-yet specified solid cancers

Source: PharmaProjects | Informa, April 2021

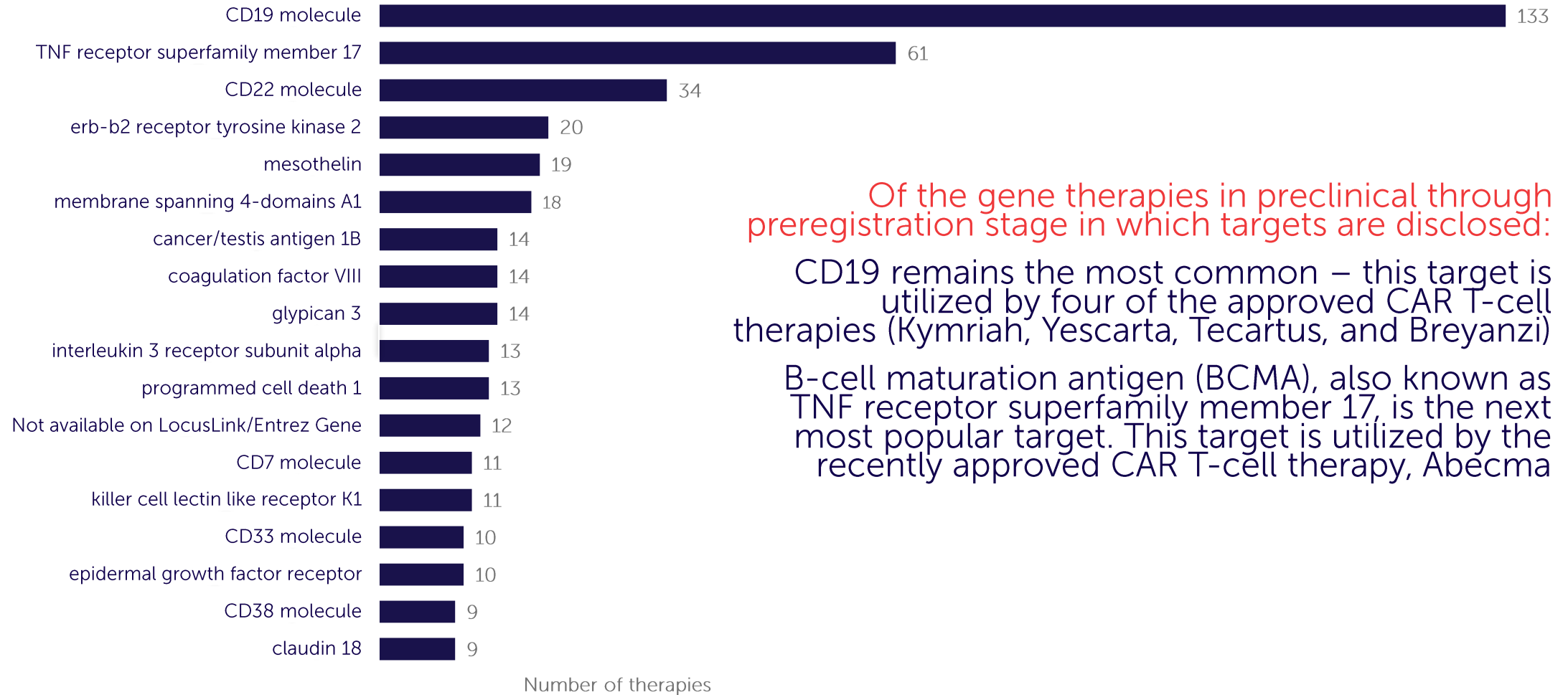
Gene therapy pipeline: Most common non-oncology diseases targeted



Neurological disease (unspecified) is the most common non-oncology disease area targeted by gene therapy, with 35 therapies in development, followed by ocular diseases (unspecified), with 29 therapies in development (preclinical to preregistration)

Source: [Pharmaprojects](#) | Informa, April 2021

Gene therapy pipeline: Most common targets



Of the gene therapies in preclinical through preregistration stage in which targets are disclosed:

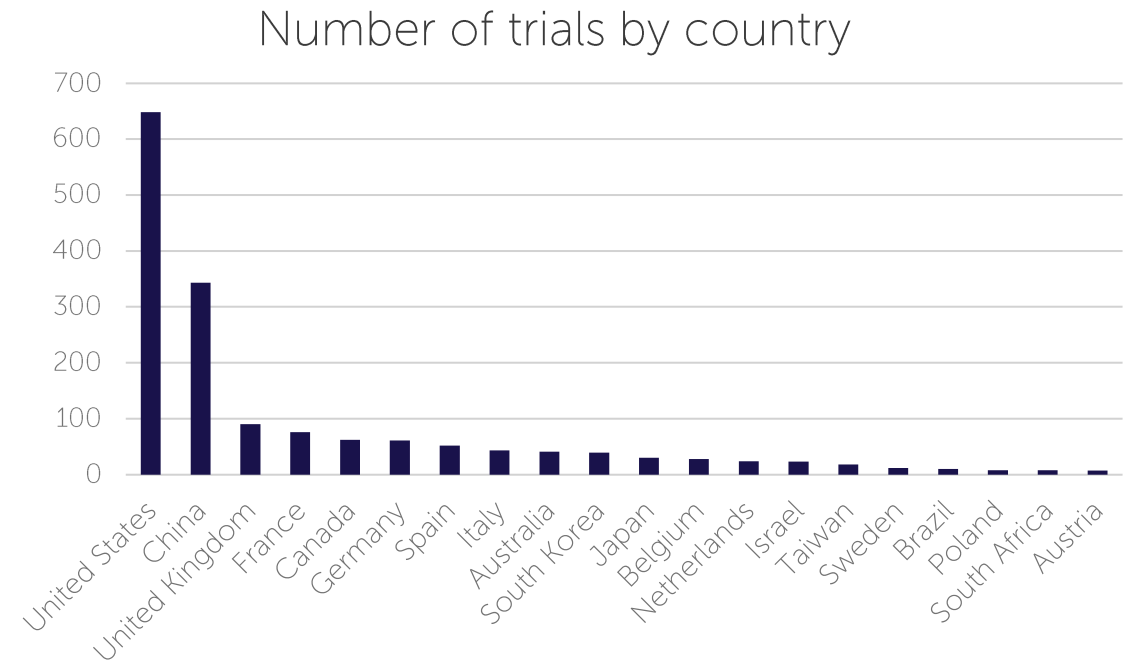
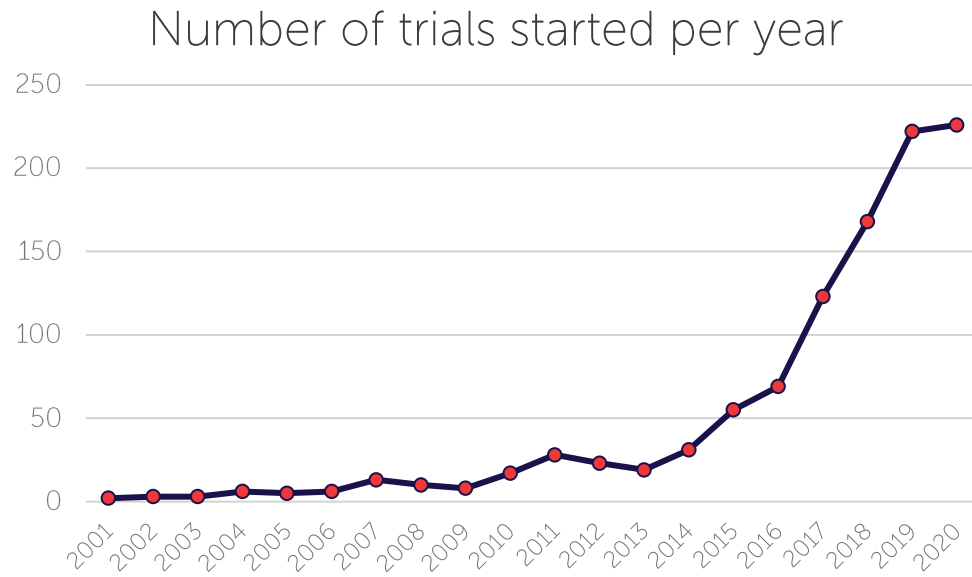
CD19 remains the most common – this target is utilized by four of the approved CAR T-cell therapies (Kymriah, Yescarta, Tecartus, and Breyanzi)

B-cell maturation antigen (BCMA), also known as TNF receptor superfamily member 17, is the next most popular target. This target is utilized by the recently approved CAR T-cell therapy, Abecma

Source: [Pharmaprojects | Informa, April 2021](#)

Gene therapy clinical trial activity

- Exponential increase in the number of gene therapy clinical trials initiated since 2013
- The United States hosts the most clinical trials for gene therapy, followed by China

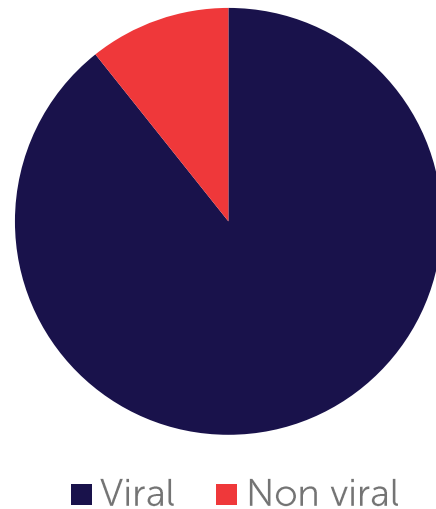


Source: [Trialtrove](#)| Informa, April 2021

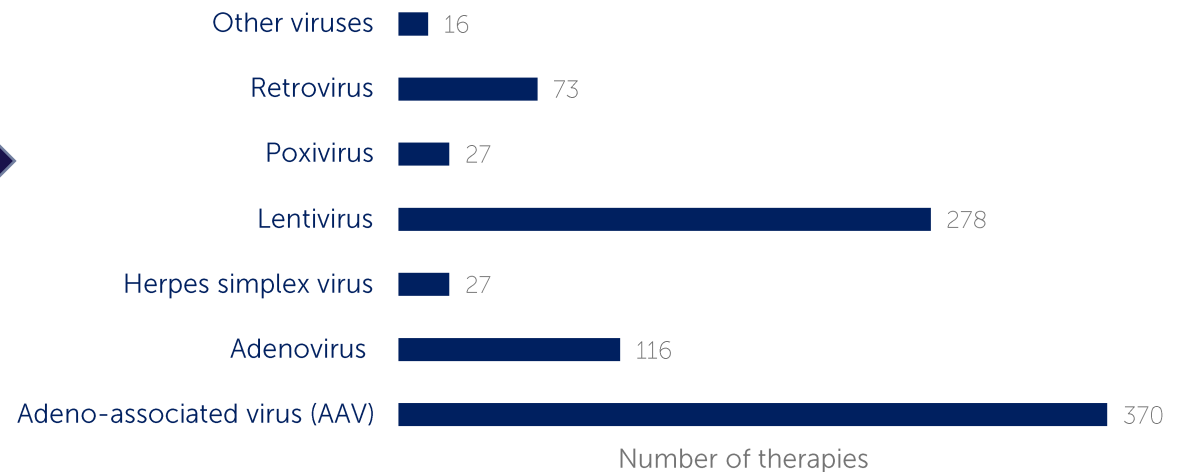
Gene therapy pipeline: Most commonly used vectors

- 88% of gene therapies in development leverage viral vectors for delivery
- Adeno-associated virus (AAV) and lentivirus are the most common viral vectors used in development

Viral vs. non-viral gene delivery



Viral vectors used in pipeline therapies



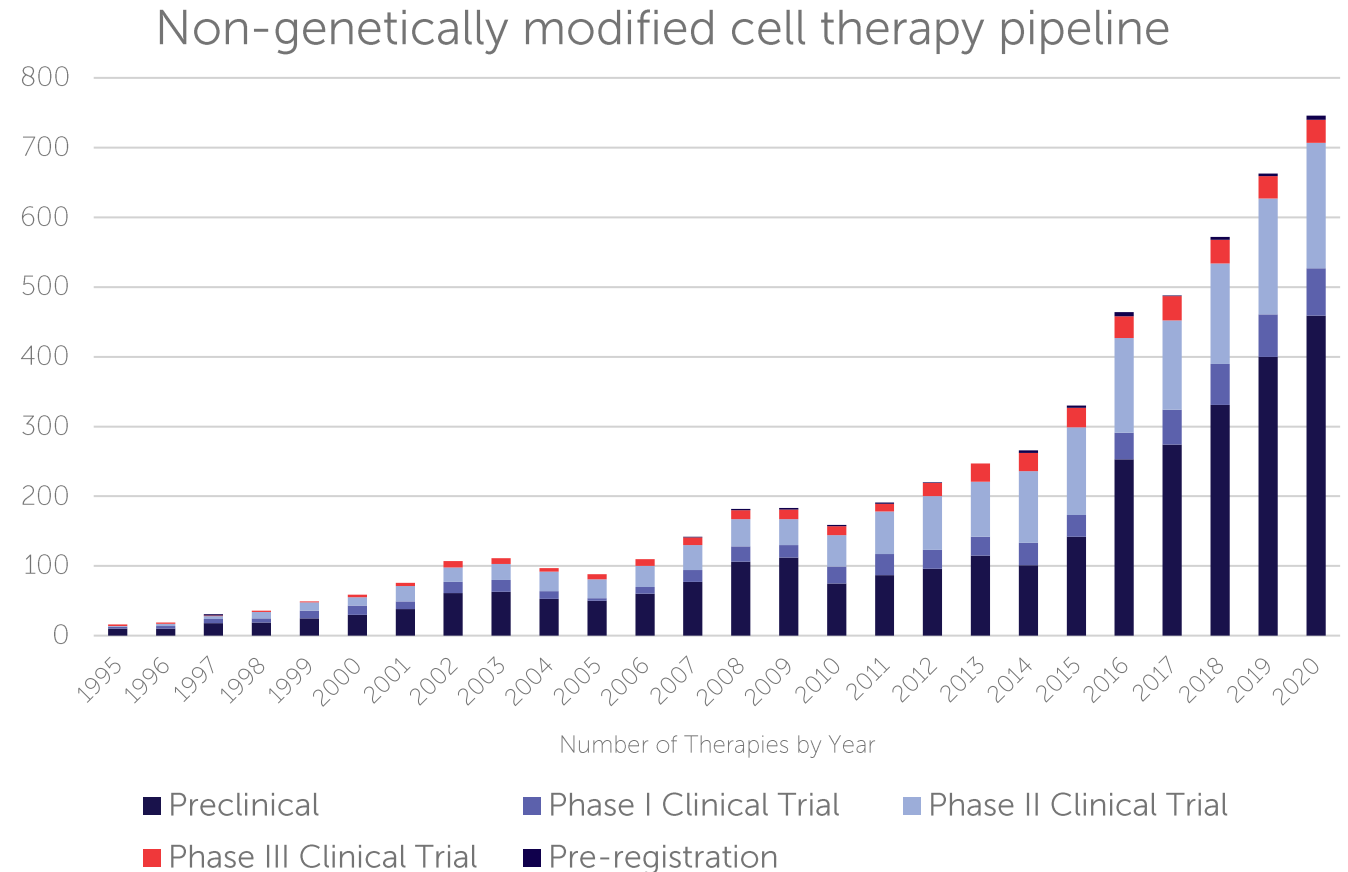
Source: Cell and Gene Therapy dashboard | Informa, April 2021

Non-genetically modified cell therapy pipeline

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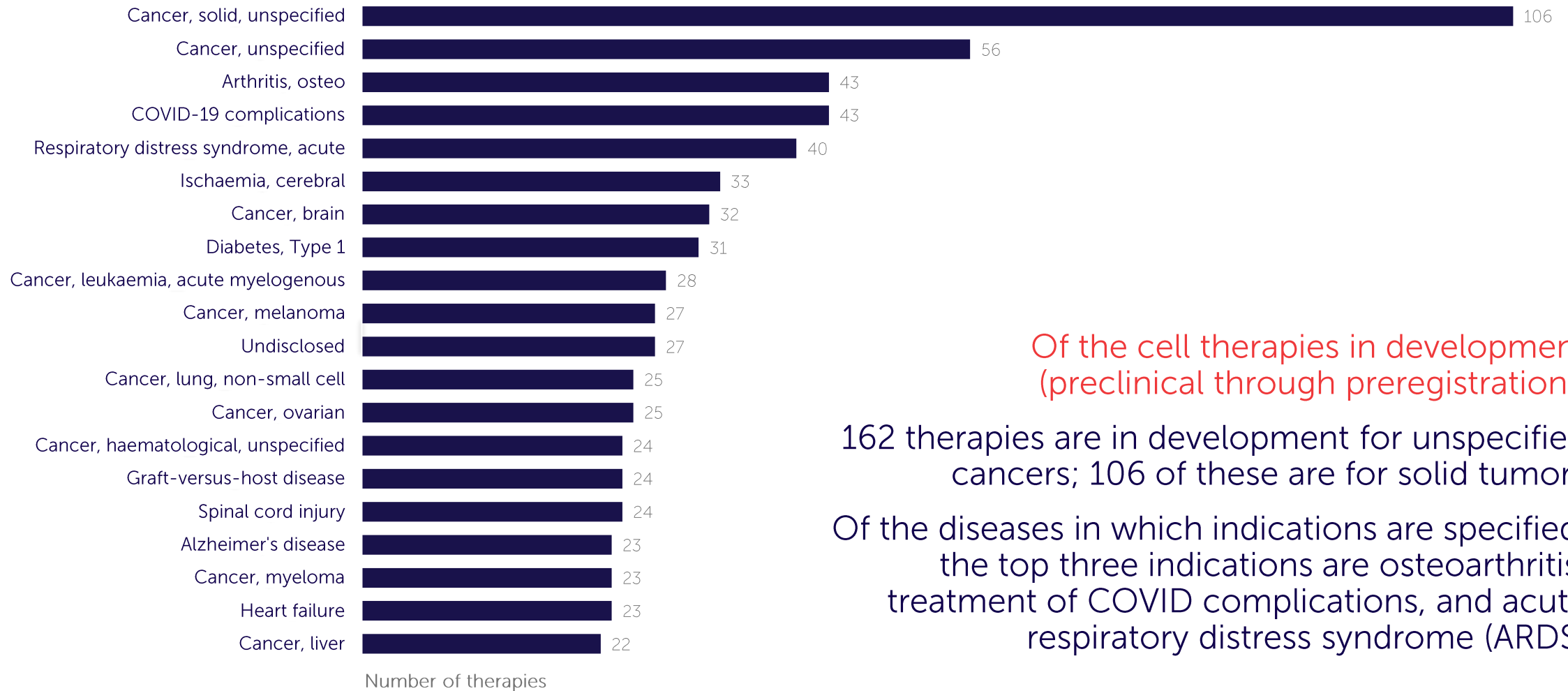
Non-genetically modified cell therapy pipeline

- The cell therapy pipeline has grown rapidly since 2015
- More than 700 cell therapies were in development in 2020 (preclinical through preregistration):
 - 61% of candidates are in preclinical development
 - There were 33 products in Phase III clinical studies



Source: [Pharmaprojects | Informa, April 2021](#)

Non-genetically modified cell therapy pipeline: Most common disease targeted



Of the cell therapies in development (preclinical through preregistration):

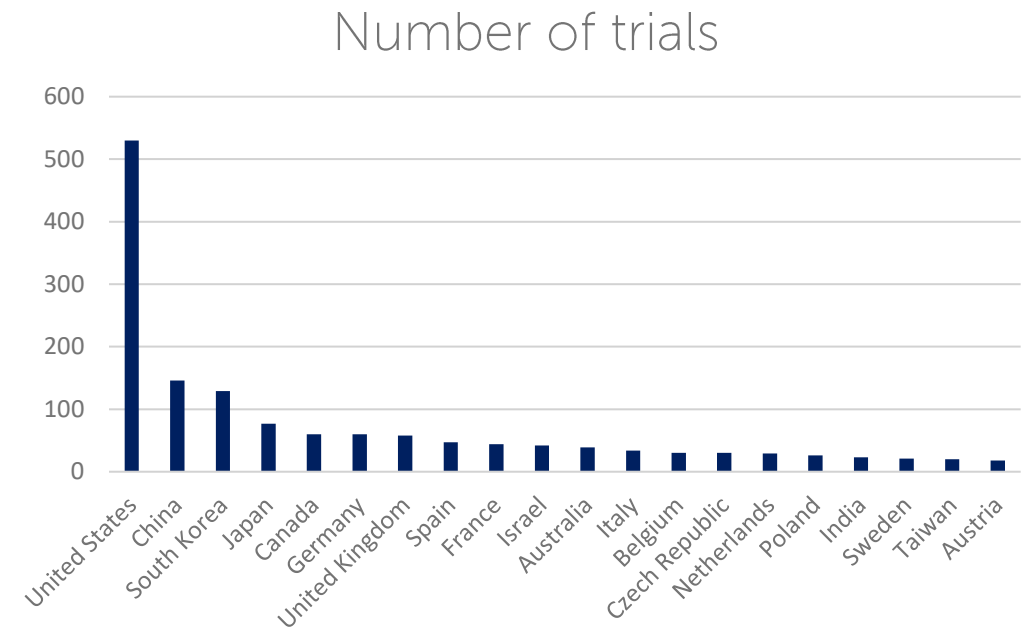
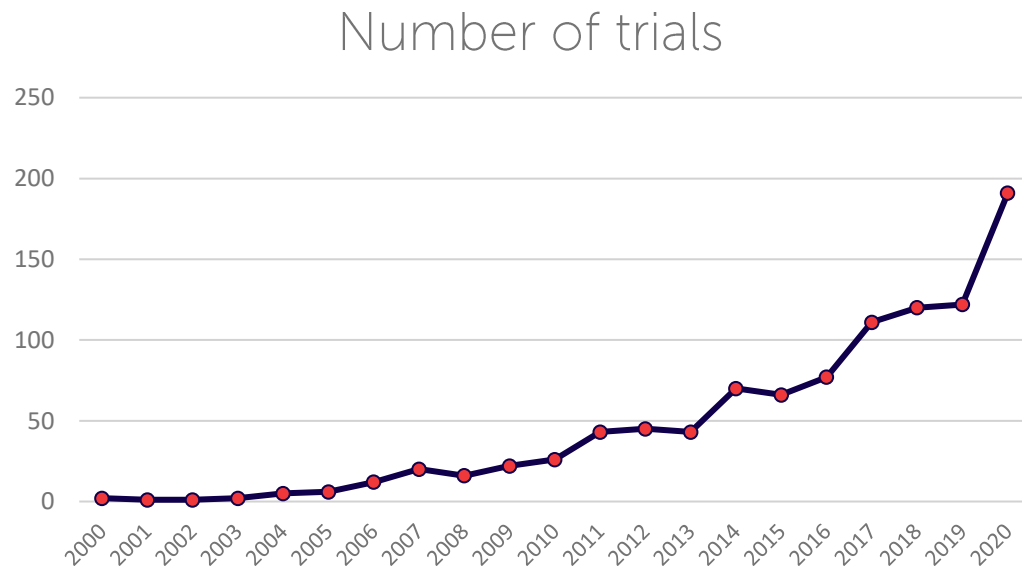
162 therapies are in development for unspecified cancers; 106 of these are for solid tumors

Of the diseases in which indications are specified, the top three indications are osteoarthritis, treatment of COVID complications, and acute respiratory distress syndrome (ARDS)

Source: Pharmaprojects| Informa, April 2021

Non-genetically modified cell therapy trial activity

- There has been an exponential increase in cell therapy clinical trials since 2013
- Trials are predominantly run in the United States, with China, South Korea, and Japan following with far fewer trials



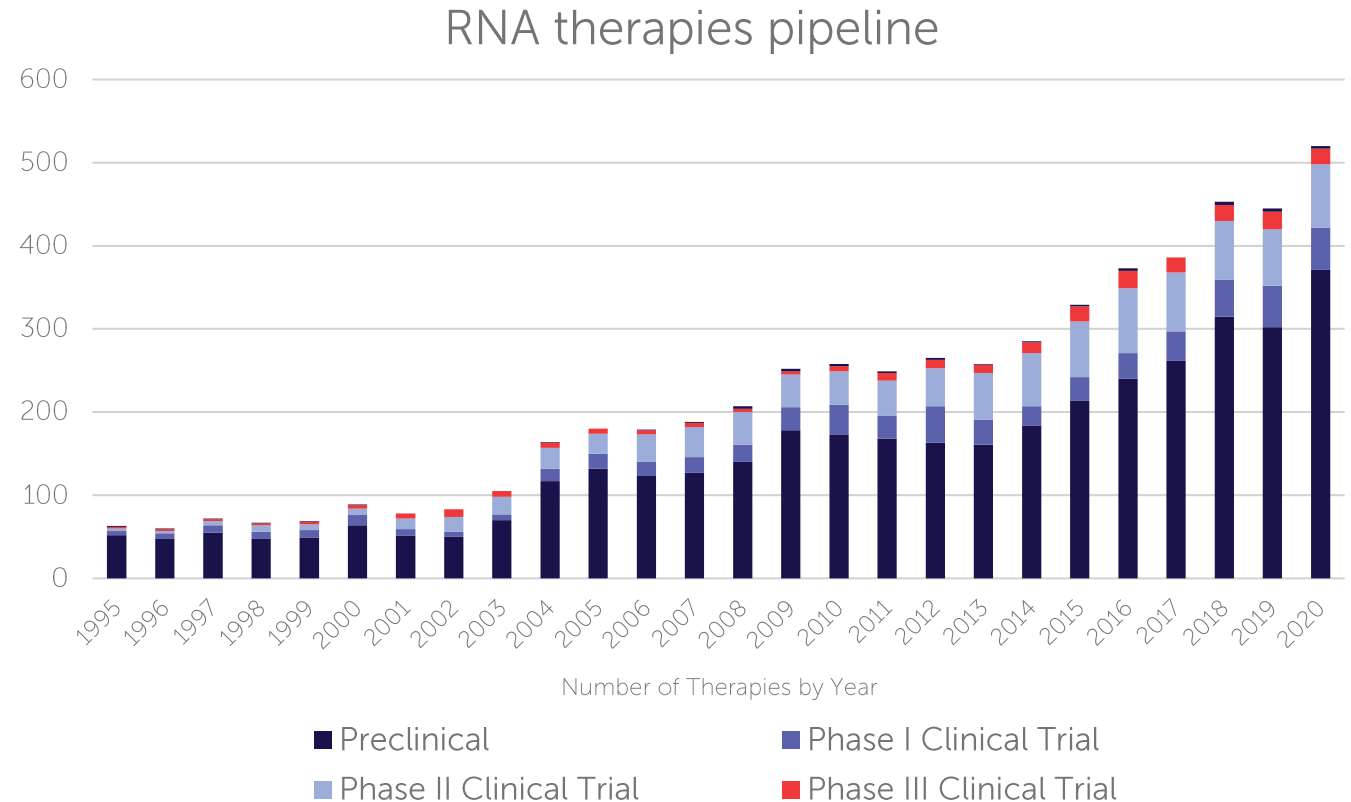
Source: [Trialtrove](#) | Informa, April 2021

RNA therapy pipeline

Q1 2021

RNA therapies pipeline

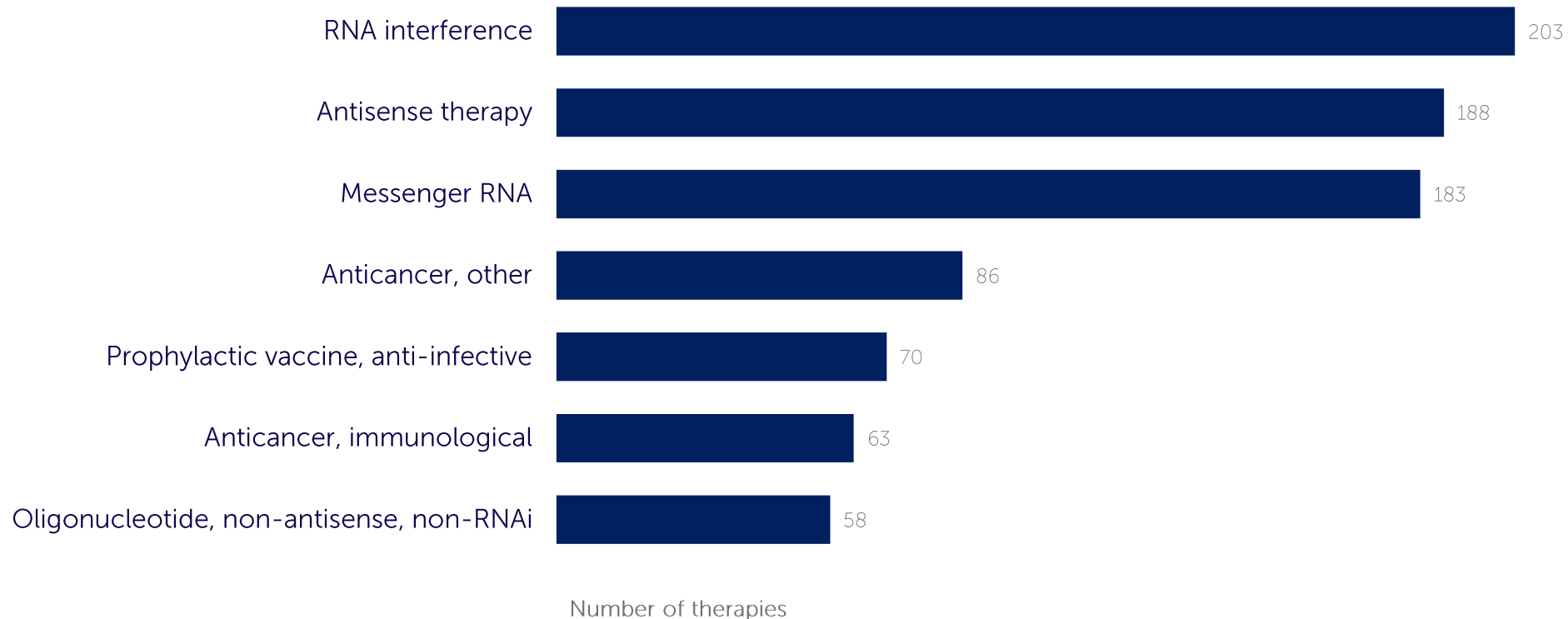
- Steady increase in RNA pipeline activity since 2004
- In 2020:
 - More than 500 therapies were in development (from preclinical to preregistration stage), with 71% of therapies in preclinical development
 - 19 therapies were in Phase III clinical studies



Source: Pharmaprojects| Informa, April 2021

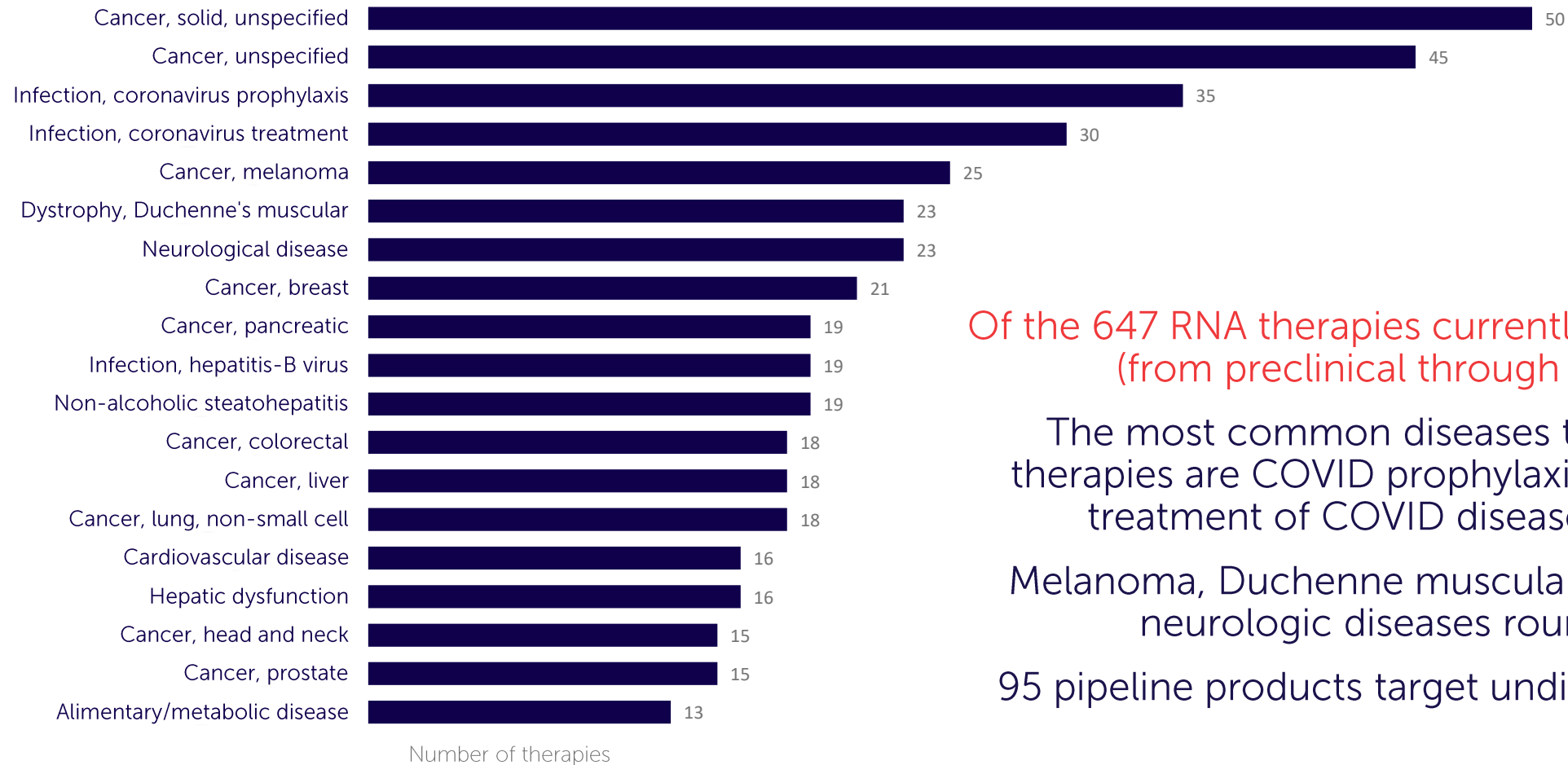
RNA therapy pipeline: Most common modalities

- The most common modalities in development (preclinical through preregistration) are RNA interference (RNAi), antisense oligonucleotides, and messenger RNA (mRNA)



Source: Pharmaprojects| Informa, April 2021

RNA therapies: Most common diseases targeted



Of the 647 RNA therapies currently in the pipeline (from preclinical through preregistration):

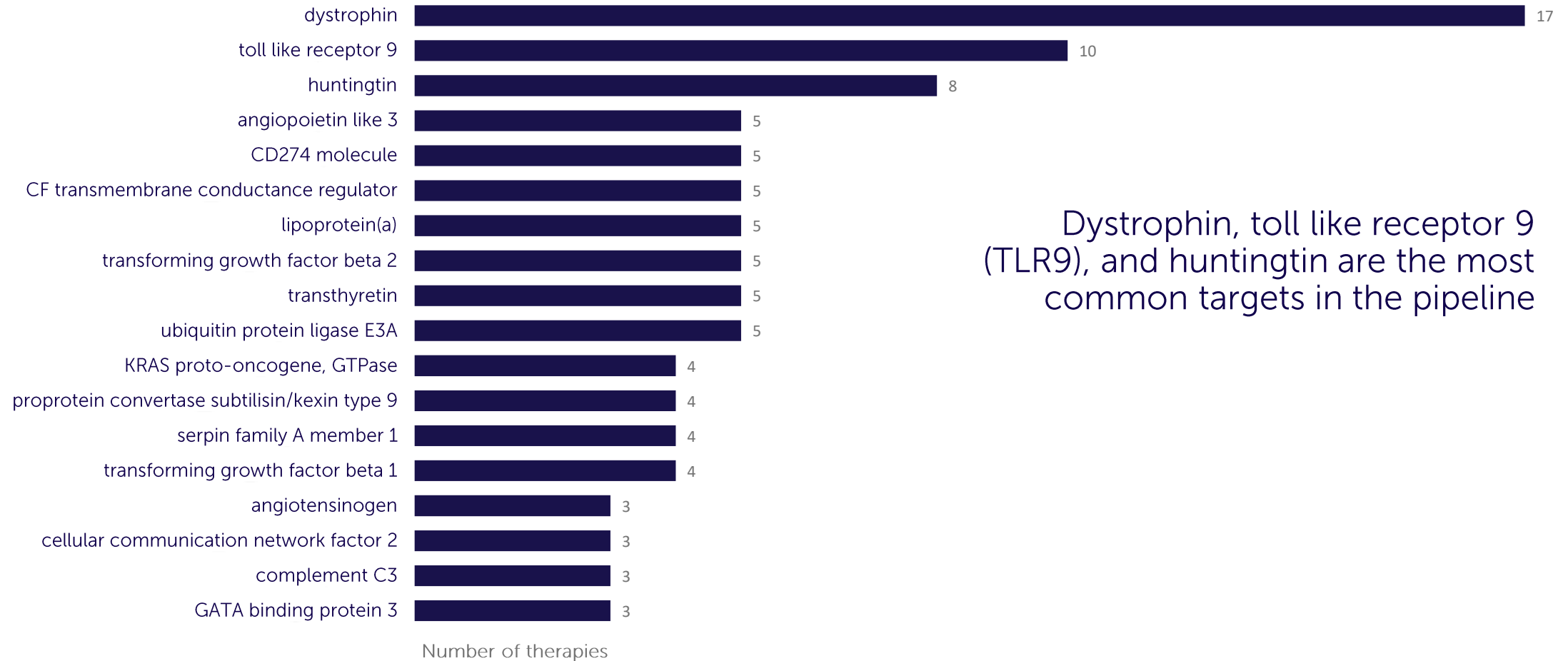
The most common diseases targeted by RNA therapies are COVID prophylaxis (vaccines) and treatment of COVID disease/complications

Melanoma, Duchenne muscular dystrophy, and neurologic diseases round out the top 5

95 pipeline products target undisclosed cancers

Source: [Pharmaprojects | Informa, April 2021](#)

RNA therapy pipeline: Most common targets



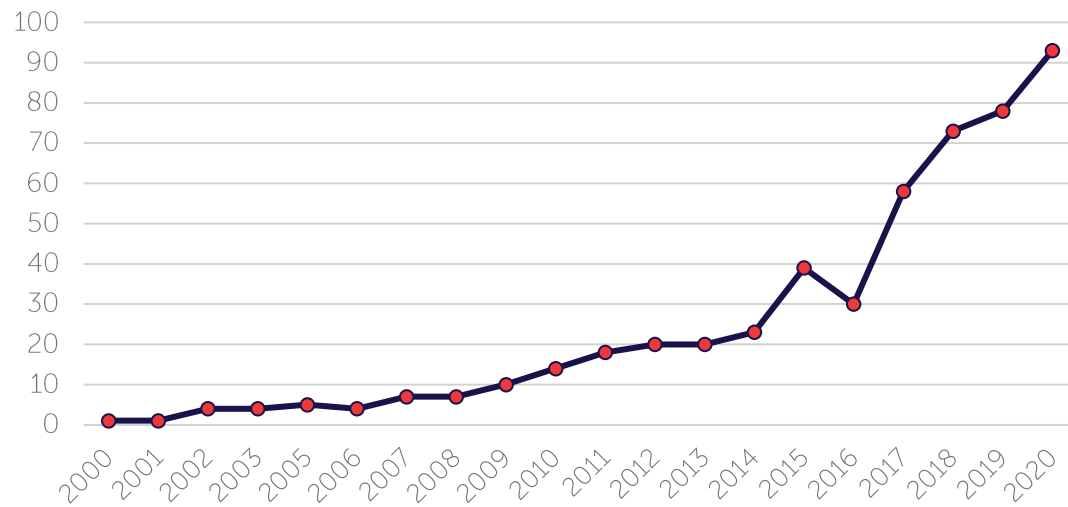
Dystrophin, toll like receptor 9 (TLR9), and huntingtin are the most common targets in the pipeline

Source: Pharmaprojects | Informa, April 2021

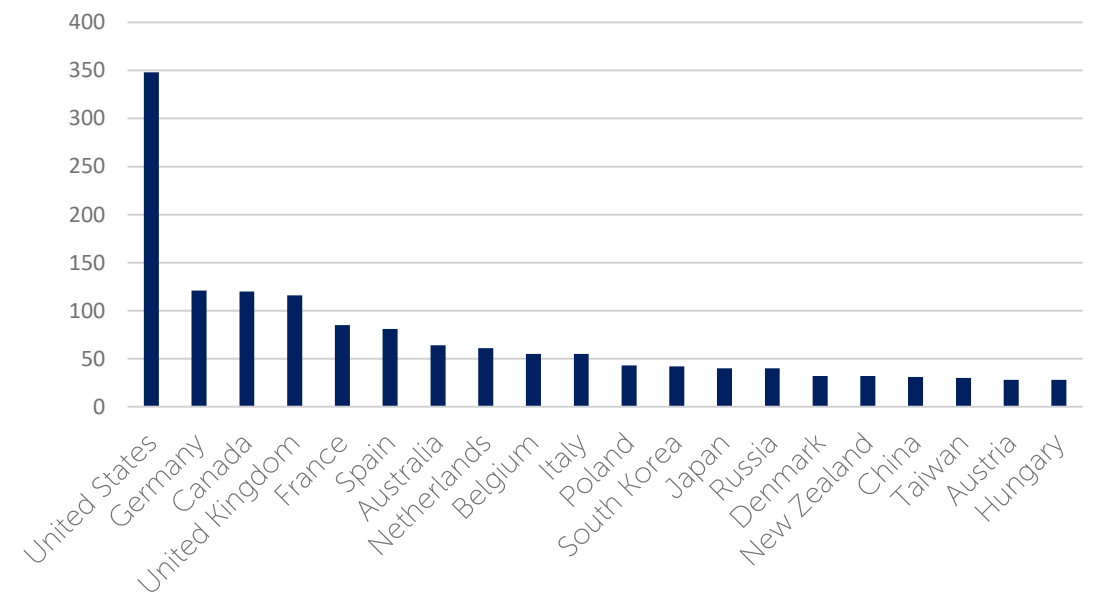
RNA therapy pipeline: Clinical trial activity

- There has been a dramatic increase in the number of RNA clinical trials started since 2016
- RNA trial activity is predominantly in the United States, followed by Germany, Canada, UK, and France

Number of trials started per year



Number of trials by country

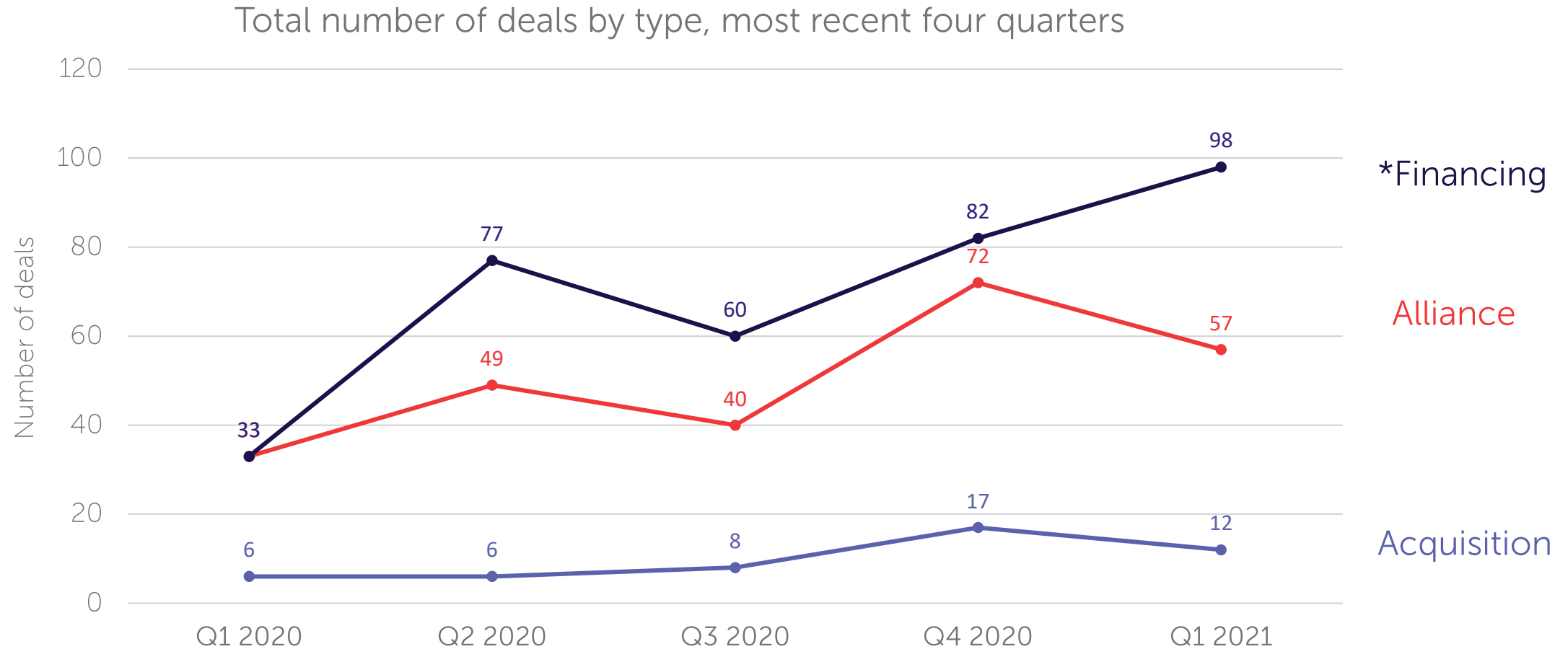


Source: [Trialtrove](#) | Informa, April 2021

Overview of dealmaking for gene, cell, and RNA therapy companies

Q1 2021

Alliance, acquisition, and financing in gene, cell, & RNA therapy



Source: Biomedtracker | Informa, April 2021

*Financings include public financings (IPOs and follow-ons) plus privately raised funding through venture rounds, debt offerings, or private investment in public equity

Q1 2021 acquisitions in gene, cell, & RNA therapy

| Deal Date | Deal Title | Potential Deal Value (USD) |
|------------|---|----------------------------|
| 01/08/2021 | Celularity Reverse Merges with Public Shell GX Acquisition Corp. to Gain Nasdaq Listing | 1,298,000,000 |
| 01/11/2021 | Bluebird bio to Separate Oncology Business into Independent Company | Undisclosed |
| 01/20/2021 | Turnstone Biologics Acquires Myst Therapeutics | Undisclosed |
| 02/03/2021 | Coya Therapeutics Completes Merger with Nicoya Health | Undisclosed |
| 02/16/2021 | Nine Biotechs Merge into Newly Formed Centessa Pharmaceuticals | Undisclosed |
| 02/17/2021 | Charles River to Acquire CDMO Cognate for \$875M | 875,000,000 |
| 02/23/2021 | Beam Therapeutics Acquires Guide Therapeutics | 440,000,000 |
| 03/02/2021 | WuXi AppTec Completes Acquisition of OXGENE | Undisclosed |
| 03/02/2021 | AgeX Therapeutics And LyGenesis To Negotiate Merger Agreement | Undisclosed |
| 03/09/2021 | Takeda Exercises Option to Acquire Maverick Therapeutics | 525,000,000 |
| 03/30/2021 | Amgen to Acquire Rodeo Therapeutics Corporation | 721,000,000 |
| 03/23/2021 | Renovacor to Reverse Merge with Public SPAC to Gain NYSE Listing | 65,000,000 |

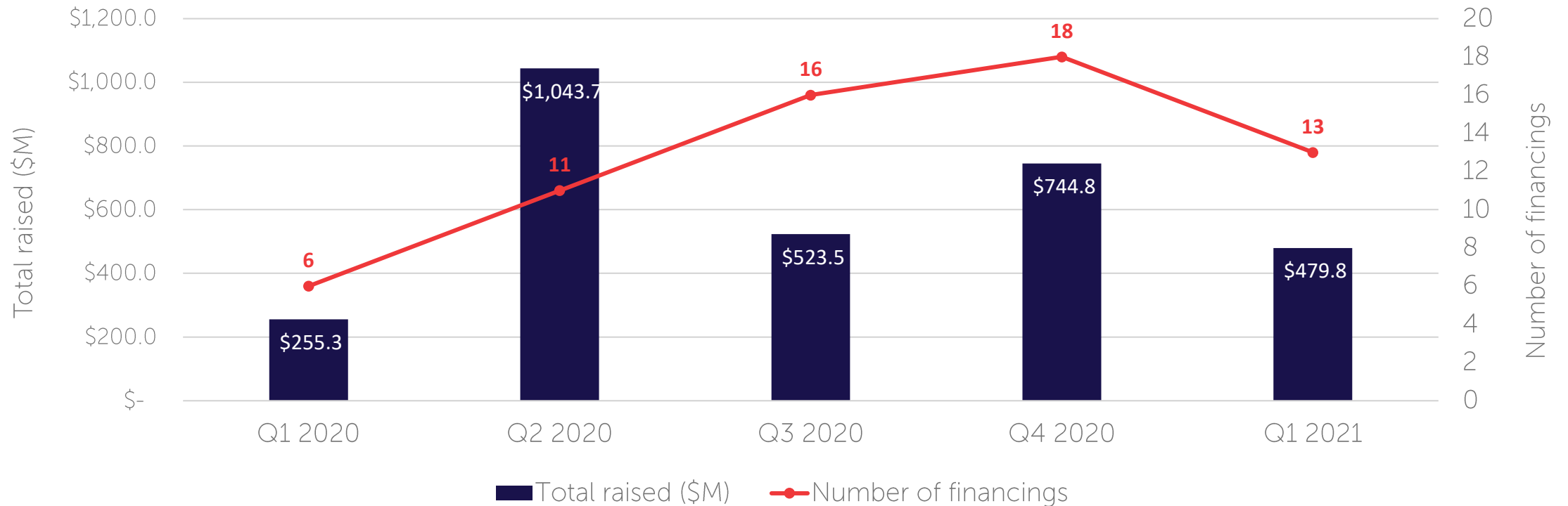
Source: Biomedtracker | Informa, April 2021

Start-up funding for gene, cell, & RNA therapy companies

Q1 2021

Start-up financing for gene, cell, & RNA therapy companies

Volume and dollar value of Series A and seed financing for gene, cell, & RNA therapy companies, most recent four quarters






Source: Biomedtracker | Informa, April 2021

Q1 2021 start-up financing for gene, cell, & RNA therapy companies

| Deal Date | Deal Title | Company Location | Academic Source | Potential Deal Value (USD) |
|------------|--|--|--|----------------------------|
| 01/06/2021 | Exacis Secures Undisclosed Seed Financing Round | United States, Massachusetts, Cambridge | n/a: Factor Biosciences spin off | Undisclosed |
| 01/07/2021 | LEXEO Therapeutics Launches with \$85M Series A | United States, New York, New York City | Weill Cornell Medicine | 85,000,000 |
| 01/08/2021 | MANA Therapeutics Launches with \$35M Series A Financing | United States, Virginia, Arlington | Children's National Hospital, Johns Hopkins Medical Center | 35,000,000 |
| 01/11/2021 | Atalanta Therapeutics Launches with Series A Financing | United States, Massachusetts, Boston | University of Massachusetts Medical School | Undisclosed |
| 02/01/2021 | AffyXell Raises \$7.3M in Series A Financing | Korea (South) | n/a: Joint venture between Avacta Group and Daewoong Pharmaceutical | 7,300,000 |
| 02/03/2021 | Coya Therapeutics Announces \$10M Series A Financing | United States, Texas, Houston | Houston Methodist Neurological Institute | 10,000,000 |
| 02/10/2021 | Notch Therapeutics Closes \$85M Series A Financing to Develop Pipeline of Renewable Stem Cell-Derived Cancer Immunotherapies | Canada, Ontario, Toronto | Sunnybrook Research Institute and University of Toronto | 85,000,000 |
| 02/11/2021 | Ensoma Launches With a \$70M Series A Financing | United States, Massachusetts, Boston | Fred Hutchinson Cancer Research Center and University of Washington School of Medicine | 70,000,000 |
| 02/24/2021 | Orna Therapeutics Launches with \$80M Series A to Develop Circular RNA Therapies | United States, Massachusetts, Cambridge | Massachusetts Institute of Technology | 80,000,000 |
| 03/01/2021 | SalioGen Therapeutics Announces Closing of \$20M Series A Financing | United States, Massachusetts, Burlington | n/a | 20,000,000 |
| 03/01/2021 | EG 427 Closes \$14.5M Series A Financing | France, Paris | SATT Paris-Saclay | 14,453,760 |
| 03/29/2021 | Qihan Biotech Raises Additional \$67M in Series A++ Financing | China, Zhejiang | n/a: affiliated with eGenesis; Harvard University co-founder | 67,000,000 |
| 03/18/2021 | Curi Bio Raises \$6M Series A Financing | United States, Washington, Seattle | University of Washington (sponsored research agreement) | 6,000,000 |

Source: Biomedtracker | Informa, April 2021

Notable Q1 2021 start-up gene, cell, & RNA therapy companies

| Company details | Academic source | Financing type/amount raised | Lead investor(s) | Therapy areas of interest |
|---|--|------------------------------|--|--|
|  <p>Engineered circular RNA therapies that address challenges with expression, delivery, and manufacturing of linear mRNA therapies</p> | Massachusetts Institute of Technology | Series A/\$80M | MPM Capital, Taiho Ventures, F2 Ventures | Oncology and autoimmune diseases, including genetic disorders |
|  <p>Exact DNA Integration Technology: Non-viral gene therapies using a mammalian-derived enzyme</p> | n/a | Series A/\$20M | PBM Capital | Cardiovascular (familial hypercholesterolemia) and ophthalmic (inherited macular degeneration) |
|  <p><i>In vivo</i> delivery of genomic modification technologies via Ingenious vectors to multiple cell types; does not require stem cell collection or myeloablative conditioning</p> | Fred Hutchinson Cancer Research Center and University of Washington School of Medicine | Series A/\$70M | 5AM Ventures | Rare diseases, plus broadly oncology, autoimmune diseases, and infectious diseases |

Source: Biomedtracker | Informa, April 2021; company websites

Appendix

Methodology, sources, & glossary of key terms

Q1 2021

Methodology: Sources and scope of therapies

Sources for all data come from Informa Pharma Intelligence

Pipeline and trial data

- Data derived from Citeline (Pharmaprojects and Trialtrove)
- Therapeutic classes included in report categorizations:
 - Gene therapies: Gene therapy; cellular therapy, chimeric antigen receptor; cellular therapy, T-cell receptor; lytic virus
 - Cell therapies: Cellular therapy, other; cellular therapy, stem cell; cellular therapy, tumor-infiltrating lymphocyte
 - RNA therapies: Messenger RNA; oligonucleotide, non-antisense, non-RNAi; RNA interference; antisense therapy

Deal and financing data

- Data derived from Biomedtracker
- The following industry categorizations of deals are included: Gene therapy, cell therapy; antisense, oligonucleotides

Glossary of key terms

Therapy type definitions

Gene therapy is the introduction, removal or change in genetic material—specifically DNA or RNA—into the cells of a patient to treat a specific disease. For the purpose of this report, the following terms shall mean the following: :

| | |
|--|---|
| Gene therapy | Therapies containing an active ingredient synthesized following vector-mediated introduction of a genetic sequence into target cells <i>in-</i> or <i>ex-vivo</i> . Used to replace defective or missing genes (as in cystic fibrosis) as well as to introduce broadly acting genetic sequences for the treatment of multifactorial diseases (e.g., cancer). Direct administration of oligonucleotides without using vectors is covered separately in the antisense therapy class; RNA interference class; or oligonucleotide, non-antisense, non-RNAi class. Platform technologies for gene delivery are covered separately in the gene delivery vector class. |
| Cellular therapy, chimeric antigen receptor <i>*Falls under gene therapy in this report</i> | Cellular therapy consisting of T cells that have been modified to express a chimeric antigen receptor (CAR) – this is a cell surface receptor that gives the T cells the ability to target a specific protein and fight the targeted cells. |
| Cellular therapy, T-cell receptor <i>*Falls under gene therapy in this report</i> | Cellular therapies whereby natural T cells collected from the patient, are engineered to express artificial receptors (usually through viral transfections) that would target specific intracellular antigens (as peptides bound to proteins encoded by the major histocompatibility complex, MHC). |
| Lytic virus <i>*Falls under gene therapy in this report</i> | Therapies which have a replication-competent virus, that lyse pathogenic cells directly. These are normally genetically modified to render them harmless to normal tissues. Examples include oncolytic viruses which specifically attack cancer cells. |

Glossary of key terms

Therapy type definitions, cont.

Cell therapy includes the following therapeutic classes:

| | |
|---|--|
| Cellular therapy, stem cell | Regenerative therapy which promotes the repair response of injured tissue using stem cells (cells from which all other specialized cells would originate). |
| Cellular therapy, tumor-infiltrating lymphocyte | Adoptive cellular transfer of tumor-resident T cells from tumor material, their expansion <i>ex vivo</i> , and transfer back into the same patient after a lymphodepleting preparative regimen. |
| Cellular therapy, other | Cellular therapies that do not fall under the categories of cellular therapy, stem cell; cellular therapy, CAR; cellular therapy, TIL; cellular therapy, TCR; or the specific cellular therapy is unspecified. |

Glossary of key terms

Therapy type definitions, cont.

RNA therapy includes the following therapeutic classes:

| | |
|--|---|
| Messenger RNA | Therapies that carry the desired mRNA code to overcome genetic mutations. The mRNA sequence will replace the defective mRNA in a patient and starts producing the desired protein. |
| Oligonucleotide, non-antisense, non-RNAi | Synthetic therapeutic oligonucleotides which operate by a mechanism other than antisense or RNA interference (RNAi). This includes ribozymes, aptamers, decoys, CpGs, and mismatched and immunostimulant oligonucleotides. Sequences delivered using vectors (gene therapy) are covered separately in "gene therapy." Antisense and RNAi oligonucleotides are covered separately in "antisense therapy" and "RNA interference," respectively. |
| RNA interference | Includes products which act therapeutically via an RNA interference (RNAi) mechanism, including small interfering RNAs (siRNAs). These may be synthetic oligonucleotides, or RNAi sequences may be expressed from a vector as a form of gene therapy (see "gene therapy" therapeutic class). <i>In vivo</i> , these sequences block the expression of a specific protein by forming an RNA-induced silencing complex, which then specifically binds to and degrades a complementary mRNA encoding the target protein. The use of RNAi purely as a drug discovery tool (e.g., in transgenic animal model production or in target validation) is not covered in this section. |
| Antisense therapy | Antisense compounds under development as potential therapeutics. These may be synthetic oligonucleotides, or antisense RNA may be expressed from a vector as a form of gene therapy. They may prevent the expression of a specific protein <i>in vivo</i> by binding to, and inhibiting the action of, mRNA, since they have a specific oligonucleotide sequence which is complementary to the DNA or RNA sequence which codes for the protein. |

Glossary of key terms

Development status definitions

| | |
|-----------------|---|
| Pipeline | Drugs that are in active development |
| Preclinical | Not yet tested in humans |
| Phase I | Early trials, usually in volunteers, safety, PK, PD |
| Phase II | First efficacy trials in small numbers of patients |
| Phase III | Large-scale trials for registrational data |
| Preregistration | Filing for approval made to regulatory authorities |
| Approved | Approval from relevant regulatory authorities for human use |

Unspecified indications

| | |
|------------------------------------|--|
| Cancer, unspecified | Indications for which the specific tumor type is not specified |
| Cancer, hematological, unspecified | Indications for which the specific hematological cancer is not specified |
| Cancer, solid, unspecified | Indications for which the specific solid tumor is not specified |

Deal type categories

| | |
|--------------|---|
| Alliances | Co-marketing, co-promotion, disease management, joint venture, manufacturing or supply, marketing-licensing, product or technology swap, product purchase, R+D and marketing-licensing, reverse licensing, trial collaborations |
| Financing | Convertible debt, FOPO, IPO, nonconvertible debt, financing/other, private investment in public equity, private placement, royalty sale, special-purpose financing vehicle, spin-off |
| Acquisitions | Buy-out, divestiture, spin-out, full acquisition, partial acquisition, reverse acquisition |



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