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Supplementary information

Genomic medicines: the coming waves?

In the format provided by the authors

Definition of genomic medicines

In this paper, we consider three categories of genomic medicines: virally delivered gene therapies, regulatory oligonucleotides (such as antisense oligonucleotides (ASOs) and siRNAs) and genome editing approaches (such as those using zinc fingers, TALENs and CRISPR systems).

We excluded mRNA-based medicines (analysed in *Nat. Rev. Drug Discov.* **20**, 735–736; 2021). At the interface with cell therapies, we only included ex vivo therapies based on genetically engineered autologous haematopoietic stem cells (for example, for sickle cell disease). Cell therapies used in immune-oncology, such as CAR-T cells, were excluded, although genome editing technologies or lentiviral-based gene delivery systems are often used to generate such therapies. We also excluded other stem cell therapies such as neural stem cell therapies, and oncolytic viruses.

Company analysis

We identified companies developing genomic medicines through a collective search of databases from Evaluate Pharma, Pitchbook and Capital IQ, as well as our own database-building from previous research. Supplementary Table 4 shows the list of companies and assets included in the analysis. The companies included should have at least one in-house genomic medicine asset or have described their major business as development of therapies focusing on genome editing, gene therapy or regulatory oligonucleotides. We also included companies without publicly disclosed clinical-stage assets. We excluded companies for which genomic medicines only account for very small portion of their business (typically large biopharma companies), and genomic medicine assets from universities, research institutes, hospitals etc. We also excluded CDMOs and CROs. However, in our programme database on which Figure 1 is based, we included all programmes in the genomic medicine space, including the pipelines from large biopharma, universities, hospitals and research institutions.

Programme analysis

We sourced the programmes in development for the above companies based on searches of the Evaluate Pharma database. In addition, we also included all genomic medicine programs we could source from large biopharma, universities, institutions, hospitals etc. We cross-checked the database with companies' websites, news reports, scientific publications and conference reports. Assets with multiple indications were counted multiple times according to the number of indications they targeted. Assets that were licensed in and out were counted only once in the database. Assets that were discontinued, disposed of or abandoned were excluded from the analysis.

We further classified the programmes into ex vivo or in vivo, the platform/tools involved (such as CRISPR, ASO or siRNA) and the delivery methods (such as lipid nanoparticle or adeno-associated virus (AAV) vector) based on public information. The overall landscape is presented in Figure 1.

Our databases for companies and programmes are as of 30 June 2022. However, there has been a wave of recent regulatory progress in the genomic medicine field, and examples of major updates are listed below. Overall, this is incrementally positive for the field, although regulatory decisions on some products have been controversial, or commercial launch and uptake have been slower than expected. These recent events do not change our overall view on the genomic medicine field.

Examples of progress with major programmes since 30 June 2022 include:

- FDA approval of bluebird bio's Zynteglo, a lentiviral-based gene therapy to treat transfusion-dependent beta-thalassaemia, on 17 August 2022.
- FDA approval of bluebird bio's Skysona, a lentiviral-based gene therapy to treat cerebral adrenoleukodystrophy, on 16 September 2022.
- FDA approval of UniQure's Hemgenix, an AAV-based gene therapy to treat haemophilia B, on 22 November 2022.
- Submission of a BLA for Sarepta's SRP-9001, an AAV-based gene therapy to treat Duchenne muscular dystrophy, in September 2022. The panel at an FDA advisory committee in May 2023 voted 8–6 in support of accelerated approval, pending full approval based on the outcome of the EMBARK trial.
- FDA accelerated approval for Biogen's Qalsody, an ASO therapy to treat SOD1-mutated amyotrophic lateral sclerosis, on 25 April 2023 based on NfL as a biomarker.
- Submission of a BLA for CRISPR Therapeutics/Vertex's CTX001, a CRISPR-mediated ex vivo genome editing therapeutic to treat sickle cell disease and transfusion-dependent beta-thalassaemia, in April 2023.
- FDA approval of Krystal Biotech's Vyjuvek, a topically delivered herpes simplex virus (HSV)-based gene therapy to treat the rare skin disorder dystrophic epidermolysis bullosa caused by the *COL7A1* mutation, on 19 May 2023.
- Resubmission of a BLA for Biomarin's Roctavian, an AAV-based gene therapy to treat haemophilia A, in September 2022. The PDUFA date is 30 June 2023.

Forecast methodology

Disease wave analysis

1. Establish the "universe" of diseases of focus

   We sourced the indication of each program through Evaluate Pharma, and cross-validated with public information, such as company websites, 10K, news and publications. We started with all diseases that have any genomic medicine with clinical activities (~270 diseases). We then excluded 1). diseases with only preclinical or discovery activities, or only abandoned activities 2). Diseases that are not well defined or disclosed in public information, such as "solid tumor" and "general respiratory disorders". These steps resulted in 119 diseases in our list (Supplementary Table 3).

2. Classified diseases into waves
   The purpose of this wave forecast is to understand when and to what extent genomic medicines for different diseases could enter the market. For this purpose, we primarily focused on timing the launch of the genomic medicine considering two factors (Supplementary Figure 2):

   - Factor 1: Scientific and technical feasibility (score 1–10)

   The scientific and technical feasibility is determined by whether a disease has known, targetable genetic mechanisms (1.1) and ease of delivery (1.2).

   1.1) Mechanism is determined by how many genes are involved simultaneously as the aetiology of the disease, and the genetic linkage of the gene mutation with the disease. Genetic disorders caused by a single and high penetration gene mutation (such as sickle cell disease) are easier to address by current genomic medicine technology than diseases caused by multiple genes, each with a mild-to moderate impact to disease onset.

   1.2) Ease of delivery is determined by the organ that should be targeted by genomic medicine. For example, the liver is the major targeted organ for haemophilia A and B. The more mature the current technology is for the delivery of the genomic medicine to the targeted organ with high specificity, the higher the score.

   - Factor 2: Market attractiveness — unmet medical needs and size of population (score 1–10).

   The market attractiveness score is determined by the unmet medical needs on a scale of 1–5 (2.1) and size of population on a scale of 0–5 (2.2).

   2.1) The unmet medical need for each disease is judged by a combination of disease severity and how well the disease can be managed by current treatment. For rare and non-cancer diseases, the severity of disease is determined by whether the disease can cause early death, severe disability, or moderate disability. For oncology indications, we assumed the disease severity is high for those where current average 5-year survival is less than 50%, and medium for those with 5-year survival of more than 50%. The effectiveness of current therapy is evaluated into "no treatment", "partial treatment", "manageable treatment" or "well managed treatment" categories. We then use a scoring matrix based on the two factors to give each disease an unmet medical need score of 1–5. The detailed scoring system is shown in Supplementary Table 3.

   2.2) The size of population is determined by both the incidence and prevalence of the diseases. For rare diseases, prevalence of disease is the primary factor. We sourced the prevalence in mature markets (US, Europe, Japan) from multiple sources (Decision Resources Group, 2021). We then adjusted for "eligible prevalence population" based on genetic features and other eligibility criteria (such as severity requirement, prior treatment etc.). For oncology, the incidence was a primary factor and the number was sourced from Decision Resources Group. For non-rare and non-oncology diseases, both prevalence and incidence were factored in. We then give a 0–5 score based on the addressable number of patients for each disease.

   - Weighting and wave determination

We considered the technology feasibility to be the most important factor for probability of success and time to succeed. Therefore, we gave a weighting of 70% to the scientific and technology score. In addition, the genomic medicine field is highly dependent on market funding for companies that are mostly "pre-revenue". Thus, market attractiveness could also serve as a positive factor to drive investment and willingness of companies to pursue research, technology development and clinical trials towards more lucrative diseases. We gave a 30% weighting on market attractiveness. We then calculated the weighted score for each disease (1–10). Based on the total score of each disease, we then classified diseases into three waves:

- Wave 1: Score >7
- Wave 2: Score 5.5–7
- Wave 3: Score <5.5

We also crosschecked our results with "Current program status", taking account of the probability of success (PoS) based on the stage of development (see table below, reference: Xie W, et al., 2021 Oct;20(10):735-736; Wong, C. H., et.al. Biostatistics 20, 273–286 (2019).; Takebe, T., et.al, Clin. Transl. Sci. 11, 597–606 (2018).) This is to factor in that if a disease has more programmes, and is at a more advanced stage (e.g., phase III), there is higher likelihood that asset will come to market earlier compared to diseases that have fewer programmes, or are at more nascent stages of development (e.g. preclinical or phase I).

PoS	**Preclinical**	**Phase I/II**	**Phase III**
Rare	8%	54%	66%
Cancer	6%	12%	49%
Non-rare	6%	25%	70%

The categorization is further calibrated with expert interviews and surveys. Hence, we adjusted about 6% of the disease categorizations to capture nuances of the disease not reflected in the weighted scoring system (Supplementary Table 3):

- Wave 1 being the near-future wave: with sufficient scientific evidence to address the disease aetiology via current technologies, abundant attempts on clinical trials (despite there being setbacks on multiple fronts that could be addressed through optimizing the current technology) and significant unmet needs. The product launch is expected in the next 1–5 years (peak sales reached in 6–10 years).
- Wave 2 is next to Wave 1, which includes diseases that have high unmet needs but with challenges to be addressed via current technology (e.g., delivery or aetiology) or limited programme supply. The product launch is expected in the next 3–10 years (peak sales reached in 8–15 years)
- Wave 3 is the final wave coming in next 5–20 years, with the most difficult diseases that need clearer knowledge of the disease aetiology or further technology development. The programme supply is limited, and most products are still in early clinical stages.

- Market size forecast

We conducted market forecast of genomic medicines for all diseases in each cohort over the next 15 years. The market value (non-risk adjusted) of each disease is determined by factors below:

1). Addressable population size:
We only included the populations of mature markets, namely USA, Germany, France, Spain, Italy, the United Kingdom and Japan. We then adjusted this population by treatment eligibility (e.g., specific requirement on genetic mutations, disease severity, age, prior treatment etc.). For rare diseases, we assumed that "warehouse" patients (prevalent population) would be treated between 1–10 years after the drug launch, and thereafter sales would primarily rely on "incidence" population. For non-rare disorders, we assumed a mix of prevalent and incident population to be treated between 1–10 years, and "incident" population only after 10 years. For oncology, we assumed only the incident population to be treated by genomic medicines.

2). Penetration of genomic medicines in addressable populations:
We assumed peak penetration to range from 5% to 25% for rare diseases, considering the level of unmet medical needs (the higher the unmet needs score is, the larger the penetration). For non-rare, or cancer indications, the

penetration ranges from 2% to 10%. The penetration assumes patient willingness to treat, affordability, and share of competitive modalities e.g., antibodies, small molecules, cell therapies etc. The penetration was obtained through a benchmark of recently launched innovative therapies in rare, non-rare/non-oncology, and oncology indications, and we further adjusted upwards the penetration, based on the assumption that genomic medicines will bring revolutionary therapeutic effects to patients, with co-payment solutions with healthcare payers from mature markets.

3). Pricing

The pricing for a certain disease is determined based on a) technology used (gene therapy, regulatory oligonucleotide, genome editing), b) number of particles (dosing) used for gene therapy only, c) disease type (rare, non-rare/non-oncology, oncology) and d) unmet medical needs. The price was determined by benchmarking the price of current marketed genomic medicines (Supplementary Table 2), and expert interviews.

4). Launch time

Expected launch time for each disease was determined by total number of existing programmes for the disease, each programme trial's status, and industry average years to next phase in the US. We also applied distribution of likelihood for early or late launches (see table below; reference: Xie W, et al., 2021 Oct;20(10):735-736; Bains, W., Drug Discovery World, 2004). Then we estimated average launch time for each wave based on clusters of launches in certain years.

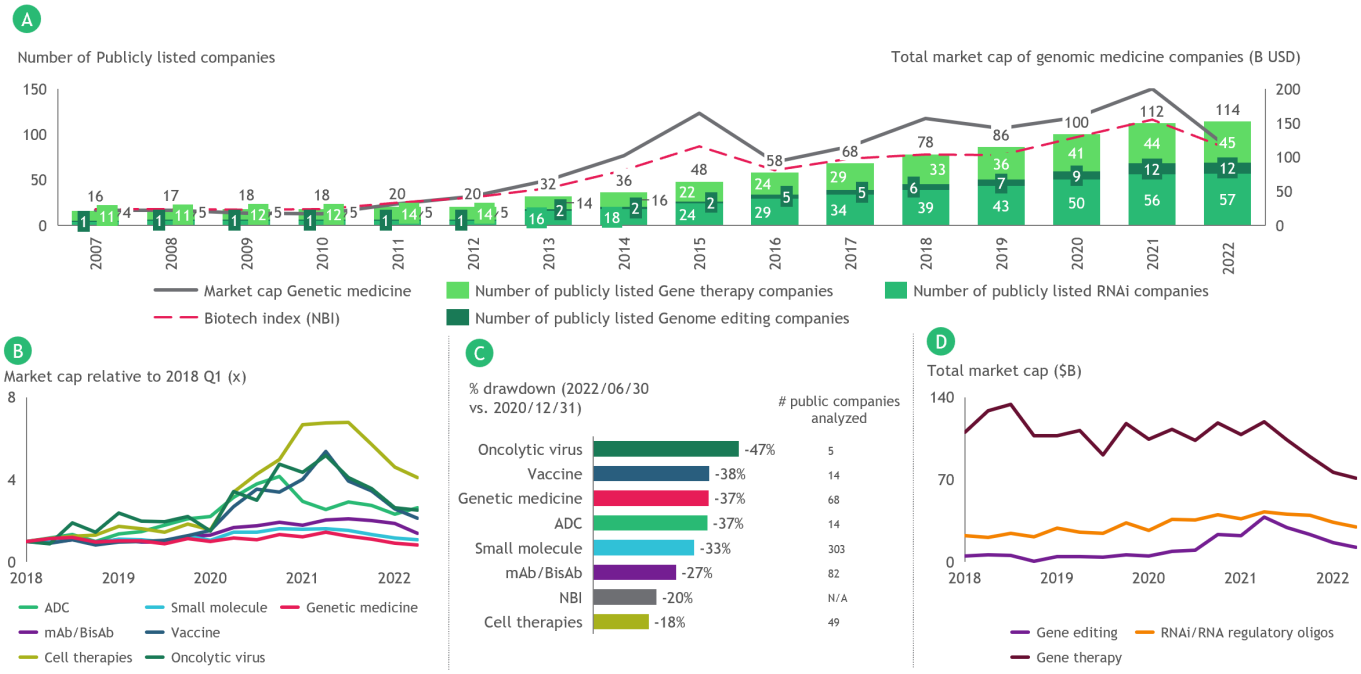
Years to Approval	Discovery	Preclinical	Phase I/II	Phase III	% of total	Total
Accel.	3	2	2	1	20%	8
Normal	4	2	4	2	60%	12
Delayed	4	3	5	3	20%	15

Expected Launch Year	Discovery	Preclinical	Phase I/II	Phase III	Marketed
Accel.	2030	2027	2025	2023	2022
Normal	2034	2030	2028	2024	2022
Delayed	2037	2033	2030	2025	2022

5). Ramp up curve

A ramp up curve was applied for each disease to reflect the gradual adoption of innovative therapies. The ramp up curve was assumed according to the disease category, namely for rare diseases (~5 year to peak), non-rare and non-oncology disorders (~7 year to peak) and oncology indications (~7 year to peak). The ramp up curve was obtained by benchmarking at least 10 typical marketed best-selling innovative therapies in each disease category.

The market value of the genomic medicine forecast result is displayed in Figure 2. Factors not directly modeled could include: patent cliff and price reduction after, probability of success for individual programmes (though it is an important factor as a "cross check" of disease wave categorization), emerging markets adoption of genomic medicine etc.



Supplementary Figure 1 | The market capitalization of publicly listed genomic medicine companies. **A** | Number of publicly listed genomic medicine companies and evolution of total market capitalization from 2007 to 2022, segmented by gene therapy, regulatory oligonucleotide and genome editing. The total market capitalization was calculated as a sum of market capitalization of all publicly listed genomic medicine companies worldwide, on 30 June 2022. The biotech index shown is NASDAQ Biotechnology Index (NBI) sourced from Refinitiv. **B** | Market capitalization relative to Q1 2018. The multiple is calculated as the total market capitalization of companies for a certain modality on 30 June 2022, relative to the total market capitalization on 31 March 2018. The company lists in each modality were sourced from Capital IQ, and manually categorized by their company description based on company official website. The companies that are focusing on multiple modalities, without in-house developed pipelines, or CRO/CDMOs were excluded. **C** | Percentage market capitalization drawdown of modalities in current economic cycle. The drawdown was calculated as the % market capitalization changes between 30 June 2022 and 31 December 2020. **D** | Total market capitalization evolution of gene therapy, genome editing and regulatory oligonucleotide companies over the past 4 years. All market capitalization data sourced from Capital IQ.

1. Feasibility Score (scale 1-10, 70% total weighting)

1.1 Known, targetable genetic mechanisms (1-5 scale)

Genetic Linkage	# Genes involved	
	Single	Multiple genes
Strong	5	4
Medium	4	3
weak	N/A	2
unclear	N/A	1

1.2 Ease of delivery (1-5 scale)

- 5: Targetable through ex vivo delivery (e.g. bone marrow), or targetable organs e.g. eye and liver delivery
- 4: Local injection or targetable organs through systemic delivery, despite moderate challenges. E.g. CNS, intra-tumor
- 3-2: Systemic injection to reachable organs. E.g. respiratory, cardiac, immune, GI, blood, dermatology
- 1: Systemic administration to difficult system. E.g. muscle

Total feasibility score= 1.1+1.2

2. Market attractiveness (scale 1-10, 30% total weighting)

2.1 Size of population (0-5 scale)

- Size sorted into categories based on US/EU5/JP prevalence
- Eligible population adjusted from US/EU5/JP prevalence and incidence
  - Adjust for "eligible prevalent population"

2.2. Unmet need (1-5 scale)

Current Treatment	Disease Severity		
	Severe	Medium	Low
No treatment	5	5	N/A
Partial treatment	4	4	2
Manageable treatment	3	2	1
Well managed	1	1	1

Total attractiveness score=2.1 + 2.2

Cohort determination

Total Score = 70% feasibility + 30% market attractiveness

Cohort1: Total score > 7

Cohort 2: Total Score 5.5 - 7

Cohort 3: Total Score <5.5

Wave categorization result was further calibrated based on:

1). Cross check with pipeline supply score:

- Calculated risk adjusted pipeline number for each diseases: to obtain a total pipeline supply score, multiplied the probability of success of certain stage with the number of pipelines in that corresponding stage.
- Diseases with highly mismatched pipeline score and wave categorization (~8% total diseases) are adjusted for wave categorization.

2). Calibrated with expert interviews to capture disease specific nuances beyond scoring (~6% total diseases)

Supplementary Figure 2 | Criteria for cohort determination of the diseases that will be cured by genomic medicines: feasibility, supply, market attractiveness.

Supplementary Table 1 | Recent development setbacks for genomic medicines

Year	Product	Disease	Stage	Event	Reason
2022	HMI-102	Phenylketonuria	Phase 1/2	Clinical trial hold (lifted 2022/06/13)	Safety concerns
2022	LB-001	Methylmalonic acidemia	Phase ½	Clinical trial hold (lifted 2022/5/9)	Safety concerns
2021	PF-06939926	Duchenne muscular dystrophy	Phase 3	Clinical trial hold (lifted 2022/4/28)	Safety concerns
2021	SEL-302	Methylmalonic acidemia	Phase ½	Clinical trial hold (lifted 2022/3/10)	Manufacturing
2021	Giroctocogene fitelparvovec	Haemophilia A	Pivotal phase 3	Clinical trial hold (lifted 2022/5/5)	Safety concerns
2021	BMN 307	Phenylketonuria	Phase ½	Clinical trial hold	Safety concerns
2021	AT132	X-linked myotubular myopathy	Phase 1/2 (ASPIRO)	Clinical trial hold (lifted 2022/4/28)	Safety concerns
2021	Elivaldogene autotemcel	Cerebral adrenoleukodystrophy	Phase 3	Clinical trial hold	Safety concerns
2021	ADVM-022	Diabetic macular oedema	Phase 2 (INFINITY)	Clinical trial fail	Safety concerns
2021	Timrepigene emparvovec	Choroideremia	Phase 3	Clinical trial fail	Missed efficacy endpoints
2021	Cotoretigene toliparvovec	X-linked retinitis pigmentosa	Phase 2/3	Clinical trial fail	Missed efficacy endpoints
2021	RP-A501	Danon disease	Phase 1	Clinical trial hold (lifted 2021/8/16)	Safety concerns
2021	WVE-120102 and WVE-120101	Huntington disease	Phase ½	Clinical trial fail	Missed efficacy endpoints
2021	Tominersen	Huntington disease	Phase ½	Clinical trial fail	Missed efficacy endpoints
2021	bb1111	Sickle cell disease	Phase ½	Clinical trial hold (lifted 2022/4/28)	Safety concerns
2020	NBIb-1817	Parkinson disease	Phase 2	Clinical trial hold	Safety concerns
2020	Etranacogene dezaparvovec (AMT-061)	Haemophilia B	Phase 3 (HOPE-B)	Clinical trial hold (lifted 2021/04/26)	Safety concerns
2020	Vocimagene amiretrorepvec (Toca511)	Glioblastoma or anaplastic astrocytoma	Phase 3	Clinical trial fail	Missed efficacy endpoints
2020	GTX-102	Angelman syndrome	Phase ½	Clinical trial hold (lifted 2021/9/27)	Safety concerns
2020	AT132	X-linked myotubular myopathy	Phase 1/2 (ASPIRO)	Clinical trial hold (lifted 2020/12/23)	Safety concerns
2020	Valrox/Roctavian	Haemophilia A	NDA	FDA Rejection of BLA application (CRL)	Durability and variability concerns
2019	SGT-001	Duchene nuscular dystrophy	Phase ½	Clinical trial hold (lifted 2020/10/1)	Safety concerns
2019	RGX-314	Wet age-related macular degeneration	Phase 2b	Clinical trial hold (lifted 2020/6/17)	Others
2019	Zolgensma	Spinal muscular atrophy type 2	Phase ½	Clinical trial hold (lifted 2021/8/3)	Safety concerns
2019	EB-101	Dystrophic epidermolysis bullosa	Phase 3	Clinical trial hold (lifted 2019/12/9)	Manufacturing
2019	PR001	Gaucher disease	Phase ½	Clinical trial hold (lifted 2020/1/6)	Others
2019	SB-913	MPS II	Phase ½	Clinical development paused	Missed efficacy endpoints
2018	SRP-9001	Duchene muscular dystrophy	Phase ½	Clinical trial hold (lifted 2018/9/24)	Manufacturing
2018	CTX001	Sickle cell disease	Phase ½	Clinical trial hold (lifted 2018/10/10)	Others

Data as of 30 June 2022.

Supplementary Table 2 | Genomic medicine products approved in the US and Europe so far

Technology class	Brand name	Generic name	Marketing company	Indication	Approved year (US)	Approved year (EU)	Annual cost ($)
Gene therapy	Glybera	Alipogene tiparvovec	uniQure	Lipoprotein lipase deficiency	-	Approved in 2012 and withdrawn in 2017	$1,000,000 one-time treatment in EU (EMA approved 2012, withdrawn in 2017)
Gene therapy	Luxturna	Voretigene neparvovec	Roche	RPE65 IRD	2017	2018	$850,000 one-time treatment
Gene therapy	Zolgensma	Onasemnogene abeparvovec	Novartis	Spinal muscular atrophy (type 1 and 2)	2019	2020	$2,125,000 one-time treatment
Gene therapy	Zynteglo	Betibeglogene autotemcel	Bluebird bio	Anaemia, sickle cell beta-thalassaemia	2022	2019	$2,800,000 one-time treatment
Gene therapy	Strimvelis		GSK	Severe combined immunodeficiency	CRL in 2018	2016	$648,000 one-time treatment (EU)
Gene therapy	Libmeldy	Atidarsagene autotemce	Orchard Therapeutics	Metachromatic leukodystrophy	-	2020	-
Gene therapy	Upstaza	Eladocagene exuparvovec	PTC Therapeutics	Aromatic L-amino acid decarboxylase (AADC) deficiency	-	2022	-
Regulatory oligonucleotide	Kynamro	Mipomersen	Sanofi	Homozygous familial hypercholesterolemia hyperlipidemia	2013	-	$348,720 per patient per year
Regulatory oligonucleotide	Exondys 51	Eteplirsen	Sarepta Therapeutics	Duchenne muscular dystrophy	2016	EPAR refused in 2018	$748,800 per year for 30-kg person
Regulatory oligonucleotide	Spinraza	Nusinersen	Biogen	Spinal muscular atrophy (type 1 and 2)	2016	2017	$750,000 first year and $375,000 subsequent years
Regulatory oligonucleotide	Onpattro	Patisiran	Sanofi	Amyloidosis, transthyretin-related	2018	2018	$450,000 per patient per year on average
Regulatory oligonucleotide	Tegsedi	Inotersen	Ionis	Amyloidosis, transthyretin-related	2018	2018	$450,000 per patient per year on average
Regulatory oligonucleotide	Vyondys 53	Golodirsen	Sarepta Therapeutics	Duchenne muscular dystrophy	2019	-	$748,800 per year for 30-kg person
Regulatory oligonucleotide	Givlaari	Givosiran	Alnylam Pharmaceuticals	Porphyria	2019	2020	$575,000 per patient per year
Regulatory oligonucleotide	Viltepso	Viltolarsen	Nippon Shinyaku	Duchenne muscular dystrophy	2020	-	$733,200 per patient per year
Regulatory oligonucleotide	Oxlumo	Lumasiran	Alnylam Pharmaceuticals	Hyperoxaluria	2020	2020	$493,000 per patient per year on average
Regulatory oligonucleotide	Amondys 45	Casimersen	Sarepta Therapeutics	Duchenne muscular dystrophy	2021	-	$748,800 per year for 30-kg person
Regulatory oligonucleotide	Leqvio	Inclisiran	Novartis	Atherosclerotic cardiovascular disease; homozygous familial hypercholesterolemia	2021	2020	$9,750 first year and $6,500 subsequent years
Regulatory oligonucleotide	Amvuttra	Vutrisiran	Alnylam Pharmaceuticals	Amyloidosis, transthyretin-related	2022	-	$463,500 per patient per year
Regulatory oligonucleotide	Waylivra	Volanesorsen	Ionis Pharmaceuticals	Familial partial lipodystrophy; lipoprotein lipase deficiency	CRL in 2018	2019	$450,000 per patient per year (EU)
Gene therapy	Lumevoq	Lenadogene nolparvovec	Gensight	Leber's hereditary optic neuropathy	-	Under review	
Gene therapy	NA	Beremagene geperpavec	Krystal Biotech	Epidermolysis bullosa	Filed (has received orphan drug designation)	Filed (received orphan drug designation)	
Gene therapy	NA	Valoctocogene roxaparvovec	Biomarin	Haemophilia A	CRL in 2020	Anticipated approval in 2023	
Gene therapy	NA	Nadofaragene firadenovec	FerGene	Bladder cancer	CRL in 2021		

We used GlobalData (https://www.globaldata.com/) to aggregate product manufacture price information and Citeline Pharmaprojects (https://citeline.informa.com) for indication and product information. Annual cost per patient was calculated based on manufacture price (USD) and usage found on public websites (e.g., product website, news website). For example, Kynamro is recommended to be given subcutaneously at a dose of 200 mg once weekly and the manufacturer's price is $7,265/200 mg in the USA. Hence, the annual cost of Kynamro per patient is $348,720 a year. Products listed without public price information are either under review or not commercially launched in the USA or the five major European markets. Products launched outside the USA or the five major European markets are not included (such as Collategene, which was approved in 2019 in Japan, indicated for peripheral artery disease). CRL, complete response letter from FDA; EPAR, European public assessment report from EMA. Data as of 30 June 2022.

Supplementary Table 3 | Full list of the indications analysed and details on scoring data

Disease	Rare/ non-rare	Wave	1. Feasibility	1.1 Known, targetable genetic mechanisms	1.2 Delivery ease	2. Attractiveness	2.1 Size of population (in ranges, in thousands, calculated based on prevalence, incidence and % eligible for genomic medicine)	2.2. Unmet medical need	Severity	Current therapy effectiveness	Program highest phase	Total programs	Total score
Achromatopsia	rare	1	9	4	5	6	5-10	4	Medium	Partial treatment	Phase I/II	4	8
Alpha-1 antitrypsin deficiency	rare	1	10	5	5	7	60-70	4	Medium	Partial treatment	Phase I/II	13	9
Amyloidosis, transthyretin-related	rare	1	10	5	5	7	40-50	4	Severe	Manageable treatment	Marketed	6	9
Amyotrophic lateral sclerosis (C9ORF)	rare	1	9	5	4	6	30-40	3	Severe	Manageable treatment	Phase I/II	6	8
Anemia, sickle cell	rare	1	9	4	5	6	120-140	3	Medium	Manageable treatment	Marketed	12	8
Aromatic L-amino acid decarboxylase deficiency	rare	1	8	4	4	6	5-10	5	Severe	No treatment	Marketed	1	8
Ataxia, spinocerebellar	rare	1	8	4	4	7	21-25	5	Severe	No treatment	Phase I/II	1	8
Autosomal recessive congenital ichthyosis	rare	1	9	5	4	5	1-5	4	Medium	Partial treatment	Phase I/II	1	8
Beta-thalassemia	rare	1	10	5	5	5	10-15	3	Medium	Manageable treatment	Marketed	12	9
Canavan disease	rare	1	9	5	4	6	<1	5	Severe	No treatment	Phase I/II	1	8
Charcot-Marie-Tooth disease	rare	1	8	4	4	6	120-140	3	Medium	Partial treatment	Phase I/II	3	8
Choroideremia	rare	1	10	5	5	5	1-5	4	Severe	Partial treatment	Phase III	2	9
Chronic granulomatous disease	rare	1	10	5	5	5	1-5	4	Medium	Partial treatment	Phase I/II	2	9
Crigler-Najjar syndrome	rare	1	10	5	5	5	1-5	4	Severe	Partial treatment	Phase I/II	3	9
Fabry disease	rare	1	10	5	5	5	1-5	4	Severe	Partial treatment	Phase I/II	11	9
Gaucher's disease, Type 1	rare	1	10	5	5	5	1-5	4	Severe	Partial treatment	Phase I/II	2	9
Hemophilia A	rare	1	10	5	5	6	30-40	3	Severe	Manageable treatment	Phase III	20	9
Hemophilia B	rare	1	10	5	5	5	5-10	3	Medium	Manageable treatment	Phase III	14	9
Hereditary angioedema	rare	1	10	5	5	5	1-5	3	Medium	Manageable treatment	Phase I/II	5	9
Hyperoxaluria	rare	1	9	4	5	5	16-20	3	Medium	Manageable treatment	Marketed	3	8
Leber's congenital amaurosis	rare	1	9	4	5	6	5-10	4	Severe	Partial treatment	Phase III	8	8
Leber's hereditary optic neuropathy	rare	1	9	4	5	6	5-10	4	Severe	Partial treatment	Phase III	3	8
Metachromatic leukodystrophy	rare	1	9	5	4	4	1-5	3	Severe	Manageable treatment	Marketed	2	8
Methylmalonic acidemia	rare	1	10	5	5	6	1-5	4	Severe	Partial treatment	Phase I/II	3	9
Mucopolysaccharidosis I (Hurler Syndrome)	rare	1	8	4	4	5	1-5	4	Severe	Partial treatment	Phase I/II	5	7
Mucopolysaccharidosis II (Hunter Syndrome)	rare	1	8	4	4	5	1-5	4	Severe	Partial treatment	Phase I/II	6	7
Mucopolysaccharidosis III (Sanfilippo Syndrome)	rare	2	8	4	4	5	1-5	4	Severe	Partial treatment	Phase III	14	7
Ornithine transcarboxylase deficiency	rare	1	10	5	5	6	10-15	4	Medium	Partial treatment	Phase I/II	1	9
Paroxysmal nocturnal hemoglobinuria	rare	1	10	5	5	5	5-10	3	Medium	Manageable treatment	Phase I/II	1	9
Phenylketonuria	rare	1	10	5	5	6	16-20	3	Medium	Manageable treatment	Phase I/II	4	9
Retinitis pigmentosa	rare	1	10	5	5	7	100-120	4	Severe	Partial treatment	Phase III	24	9
RPE-65 IRD	rare	1	10	5	5	4	1-5	3	Severe	Manageable treatment	Marketed	4	9
Severe combined immunodeficiency	rare	1	9	4	5	4	<1	3	Severe	Manageable treatment	Marketed	5	8

Spinal muscular atrophy (type 1 and 2)	rare	1	9	5	4	5	21-25	3	Severe	Manageable treatment	Marketed	2	8
Wilson's disease	rare	1	10	5	5	5	30-40	3	Severe	Manageable treatment	Phase I/II	4	9
Wiskott-Aldrich syndrome	rare	1	10	5	5	5	<1	4	Severe	Partial treatment	Phase III	2	9
Usher syndrome	rare	1	5	4	1	6	26-30	4	Severe	Partial treatment	Phase III	2	5
Atherosclerosis	non-rare	2	7	3	4	6	1000-2000	1	Medium	Well managed treatment	Marketed	4	7
Hyperlipidaemia	non-rare	2	8	3	5	6	2000-3000	1	Low	Manageable treatment	Marketed	10	8
Infection, hepatitis-B virus	non-rare	2	8	3	5	6	2000-3000	2	Low	Partial treatment	Phase I/II	19	8
Hepatoma, liver cancer	cancer	2	8	3	5	8	21-25	4	Severe	Partial treatment	Phase I/II	14	8
Acromegaly	rare	2	6	2	4	6	10-15	3	Medium	Manageable treatment	Phase I/II	2	6
Adrenal hyperplasia, congenital	rare	2	7	5	2	7	21-25	4	Medium	Partial treatment	Phase I/II	1	7
Cystic fibrosis	rare	2	6	5	1	6	30-40	3	Medium	Manageable treatment	Phase I/II	19	6
Cystinosis	rare	2	7	5	2	5	1-5	4	Severe	Partial treatment	Phase I/II	1	7
Duchenne Muscular Dystrophy	rare	2	6	5	1	5	16-20	3	Severe	Manageable treatment	Marketed	36	6
Homozygous familial hypercholesterolemia	rare	2	9	4	5	4	1-5	3	Medium	Manageable treatment	Marketed	7	8
Huntington's disease	rare	2	9	5	4	7	26-30	4	Severe	Partial treatment	Phase I/II	3	9
Pompe's disease	rare	2	6	5	1	6	1-5	4	Severe	Partial treatment	Phase I/II	10	6
Cardiovascular disease with high Lp(a)	non-rare	2	8	3	5	8	6000-7000	3	Medium	Manageable treatment	Phase I/II	1	8
Chronic kidney disease (CKD)	non-rare	3	4	2	2	8	4000-5000	3	Medium	Manageable treatment	Phase I/II	3	5
Glaucoma	non-rare	2	6	2	4	6	3000-4000	1	Severe	Well managed treatment	Phase I/II	5	6
IgA nephropathy	non-rare	2	7	3	4	7	160-180	3	Medium	Manageable treatment	Phase I/II	2	7
Macular degeneration (various causes)	non-rare	2	7	2	5	8	>10000	3	Severe	Manageable treatment	Phase III	30	7
Macular edema	non-rare	2	8	3	5	7	1000-2000	3	Severe	Manageable treatment	Phase III	2	8
Non-alcoholic Steatohepatitis	non-rare	2	6	1	5	9	2000-3000	4	Medium	Partial treatment	Phase I/II	8	7
Parkinson's disease	non-rare	2	6	2	4	8	1000-2000	4	Severe	Partial treatment	Marketed	15	7
Coronary artery disease	non-rare	2	5	2	3	8	350-400	3	Medium	Manageable treatment	Marketed	3	6
Epidermolysis Bullosa	rare	2	5	3	2	5	5-10	4	Severe	Partial treatment	Marketed	14	5
Overactive bladder	non-rare	3	4	2	2	8	5000-6000	3	Medium	Manageable treatment	Phase I/II	1	5
Basal cell carcinoma	cancer	3	3	2	1	8	180-200	3	Medium	Manageable treatment	Phase I/II	2	4
Melanoma	cancer	2	6	3	3	6	16-20	3	Medium	Manageable treatment	Phase III	10	6
Mucopolysaccharidosis VI (Maroteaux-Lamy Syndrome)	rare	2	6	5	1	6	<1	5	Severe	No treatment	Phase I/II	1	6
Dystrophy, limb-girdle muscular	rare	3	4	3	1	6	1-5	4	Medium	Partial treatment	Phase I/II	10	4
Dystrophy, myotonic muscular	rare	3	5	4	1	5	1-5	4	Medium	Partial treatment	Phase I/II	7	5
Familial adenomatous polyposis	rare	2	7	5	2	6	1-5	3	Severe	Manageable treatment	Phase I/II	1	7
Giant Axonal neuropathy	rare	3	9	5	4	4	<1	4	Medium	Partial treatment	Phase I/II	1	8
GM1 gangliosidosis	rare	3	9	5	4	5	<1	5	Severe	No treatment	Phase I/II	1	8
Hypertrophic Scarring	rare	3	6	2	4	3	<1	2	Low	Partial treatment	Phase I/II	1	5
Lipoprotein lipase deficiency	rare	3	5	4	1	4	<1	3	Medium	Manageable treatment	Marketed	2	5
Myasthenia gravis	rare	3	3	2	1	6	100-120	3	Severe	Manageable treatment	Phase I/II	1	4
Myelodysplastic syndrome	rare	3	4	2	2	7	80-90	4	Medium	Partial treatment	Phase I/II	2	5
Porphyria	rare	3	4	3	1	8	30-40	5	Medium	Partial treatment	Marketed	1	5
Pulmonary fibrosis, idiopathic	rare	3	4	2	2	6	30-40	3	Medium	Manageable treatment	Phase I/II	5	4

Scleroderma	rare	3	3	2	1	6	<1	3	Medium	Manageable treatment	Phase I/II	2	4
Tay-Sachs	rare	3	9	5	4	4	<1	4	Severe	Partial treatment	Phase I/II	3	8
Anemia, aplastic, Fanconi's	rare	3	6	4	2	5	1-5	4	Severe	Partial treatment	Phase I/II	4	5
Lumbosacral Radiculopathy	non-rare	2	5	1	4	9	1000-2000	4	Medium	Partial treatment	Phase I/II	1	6
Neuropathy, diabetic	non-rare	2	7	2	5	7	2000-3000	2	Medium	Manageable treatment	Phase III	1	7
Retinopathy, diabetic	non-rare	2	7	2	5	7	7000-8000	2	Medium	Manageable treatment	Phase I/II	4	7
Alzheimer's disease	non-rare	3	6	2	4	8	5000-6000	4	Severe	Partial treatment	Phase I/II	3	6
Arthritis, rheumatoid	non-rare	3	2	1	1	5	350-400	1	Medium	Well managed treatment	Phase I/II	7	3
Benign prostatic hyperplasia	non-rare	3	4	3	1	8	800-850	3	Medium	Manageable treatment	Phase I/II	1	5
Claudication	non-rare	3	3	2	1	7	4000-5000	3	Medium	Manageable treatment	Phase I/II	1	4
Crohn's disease	non-rare	3	4	2	2	7	800-850	3	Severe	Manageable treatment	Phase I/II	3	5
Eczema/Dermatitis	non-rare	3	3	1	2	8	>10000	3	Medium	Manageable treatment	Phase I/II	2	4
Fibrosis, liver	non-rare	2	7	2	5	9	5000-6000	4	Severe	Partial treatment	Phase I/II	4	7
Foot ulcers, diabetic	non-rare	3	2	1	1	8	>10000	3	Medium	Manageable treatment	Phase III	2	3
Genetic hearing loss	non-rare	3	7	3	4	8	1000-2000	4	Severe	Partial treatment	Phase I/II	12	7
Gout	non-rare	3	4	2	2	6	2000-3000	1	Low	Manageable treatment	Phase I/II	2	4
Heart failure	non-rare	2	5	2	3	6	3000-4000	1	Severe	Well managed treatment	Phase III	7	5
Infection, HIV	non-rare	3	4	3	1	5	300-350	1	Severe	Well managed treatment	Phase I/II	11	4
Infection, human papilloma virus	non-rare	3	3	2	1	8	1000-2000	3	Medium	Manageable treatment	Phase I/II	8	4
Infection, RSV	non-rare	3	4	2	2	8	26-30	4	Medium	Partial treatment	Phase I/II	2	5
Peripheral arterial disease	non-rare	3	4	2	2	8	6000-7000	3	Medium	Manageable treatment	Marketed	5	5
Pouchitis	non-rare	3	3	1	2	7	400-450	3	Medium	Manageable treatment	Phase III	1	4
Sjogren's syndrome	non-rare	3	4	3	1	8	160-180	4	Medium	Partial treatment	Phase I/II	1	4
Tendinitis & Bursitis	non-rare	3	3	1	2	7	200-250	3	Medium	Manageable treatment	Phase I/II	1	4
Brain cancer	cancer	2	5	2	3	8	10-15	4	Severe	Partial treatment	Phase III	14	6
Leukemia, acute myeloid	cancer	3	3	1	2	6	5-10	3	Severe	Manageable treatment	Phase I/II	2	4
Leukemia, chronic lymphocytic	cancer	3	3	1	2	8	1-5	3	Severe	Manageable treatment	Phase I/II	2	4
Mesothelioma	cancer	3	3	2	1	8	1-5	4	Severe	Partial treatment	Phase I/II	1	4
Prostate cancer	cancer	3	3	1	2	8	250-300	4	Medium	Partial treatment	Phase III	10	4
Skin cancer, non-melanoma	cancer	2	5	2	3	8	350-400	3	Medium	Manageable treatment	Phase I/II	4	6
Thyroid cancer	cancer	3	4	2	2	5	<1	3	Medium	Manageable treatment	Phase III	3	4
Bladder cancer	cancer	3	3	1	2	7	40-50	3	Severe	Manageable treatment	Phase III	4	4
Bone cancer (Osteosarcoma)	cancer	3	6	2	4	9	<1	4	Severe	Partial treatment	Phase I/II	2	7
Breast cancer	cancer	3	4	2	2	7	100-120	3	Medium	Manageable treatment	Phase I/II	8	5
Colorectal cancer	cancer	3	4	2	2	8	90-100	4	Severe	Partial treatment	Phase I/II	4	5
cutaneous squamous cell carcinoma	cancer	3	4	2	2	7	5-10	3	Medium	Manageable treatment	Phase I/II	1	5
Head & neck cancers	cancer	3	7	3	4	6	50-60	3	Severe	Manageable treatment	Phase III	11	7
Multiple myeloma	cancer	3	3	1	2	7	5-10	4	Severe	Partial treatment	Phase I/II	1	4
Neuroendocrine tumor	cancer	3	3	2	1	7	30-40	3	Medium	Manageable treatment	Phase III	1	4
Non-Hodgkin lymphoma	cancer	3	3	2	1	7	40-50	3	Medium	Manageable treatment	Phase I/II	2	4
Non-small cell lung cancer	cancer	3	4	2	2	7	100-120	3	Severe	Manageable treatment	Phase III	10	5
Ovarian cancer	cancer	2	5	3	2	7	26-30	3	Severe	Manageable treatment	Phase III	5	5
Pancreatic cancer	cancer	3	4	2	2	7	70-80	5	Severe	No Treatment	Phase I/II	5	5
Renal cell carcinoma	cancer	3	4	2	2	7	1-5	4	Severe	Partial treatment	Phase I/II	1	5

Supplementary Table 4 | List of companies and programmes included in the analysis

Name of company	Major category	# Discovery/ preclinical programmes	# Clinical-stage programmes	# Marketed programmes
Sirnaomics	Regulatory oligonucleotide	17	10	0
Sarepta Therapeutics	Gene therapy	17	6	1
Ionis Pharmaceuticals	Regulatory oligonucleotide	4	15	2
Alnylam Pharmaceuticals	Regulatory oligonucleotide	9	9	2
ProQR Therapeutics	Regulatory oligonucleotide	15	4	0
Arrowhead Pharmaceuticals	Regulatory oligonucleotide	7	8	0
Editas Medicine	Genome editing	12	2	0
Généthon	Gene therapy	7	6	0
Sangamo Therapeutics	Genome editing	8	5	0
Beam Therapeutics	Genome editing	12	0	0
uniQure	Regulatory oligonucleotide	7	4	1
InteRNA Technologies	Regulatory oligonucleotide	7	4	0
Krystal Biotech	Gene therapy	8	2	1
OliPass	Regulatory oligonucleotide	10	1	0
Orchard Therapeutics	Gene therapy	5	5	1
Isarna Therapeutics	Regulatory oligonucleotide	8	2	0
AnGes	Regulatory oligonucleotide	9	0	0
Helixmith	Gene therapy	3	6	0
M6P Therapeutics	Gene therapy	9	0	0
Phio Pharmaceuticals	Regulatory oligonucleotide	9	0	0
REGENXBIO	Gene therapy	2	7	0
Applied Genetic Technologies	Gene therapy	4	4	0
Deep Genomics	Regulatory oligonucleotide	8	0	0
OliX Pharmaceuticals	Regulatory oligonucleotide	7	1	0
Precigen	Gene therapy	3	5	0
Silence Therapeutics	Regulatory oligonucleotide	4	4	0
Intellia Therapeutics	Genome editing	4	3	0
ToolGen	Genome editing	7	0	0
4D Molecular Therapeutics	Gene therapy	4	2	0
Adverum Biotechnologies	Gene therapy	3	3	0
Avidity Biosciences	Regulatory oligonucleotide	5	1	0
BridgeBio Pharma	Gene therapy	3	3	0
Fortress Biotech	Gene therapy	4	2	0
Generation Bio	Gene therapy	6	0	0
LogicBio Therapeutics	Gene therapy	5	1	0
MeiraGTx	Gene therapy	2	4	0
Rocket Pharmaceuticals	Gene therapy	0	6	0
Ultragenyx Pharmaceutical	Gene therapy	2	4	0
Abeona Therapeutics	Gene therapy	2	3	0
ASC Therapeutics	Genome editing	3	2	0
Benitec Biopharma	Regulatory oligonucleotide	4	1	0

Decibel Therapeutics	Gene therapy	3	2	0
Homology Medicines	Gene therapy	2	3	0
IVERIC bio	Gene therapy	5	0	0
Akouos	Gene therapy	4	0	0
American Gene Technologies	Gene therapy	3	1	0
Amicus Therapeutics	Gene therapy	4	0	0
Arbutus Biopharma	Regulatory oligonucleotide	3	1	0
AVROBIO	Gene therapy	1	3	0
BioMarin Pharmaceutical	Gene therapy	1	3	0
Candel Therapeutics	Gene therapy	0	4	0
Celsion	Gene therapy	2	2	0
CRISPR Therapeutics	Genome editing	3	1	0
CSL	Gene therapy	2	2	0
Gene Signal	Regulatory oligonucleotide	1	3	0
GenSight Biologics	Gene therapy	2	2	0
Gradalis	Regulatory oligonucleotide	2	2	0
PsiOxus Therapeutics	Gene therapy	2	2	0
Renova Therapeutics	Gene therapy	4	0	0
Selecta Biosciences	Gene therapy	4	0	0
Sio Gene Therapies	Gene therapy	0	4	0
Symvivo	Gene therapy	3	1	0
Vir Biotechnology	Regulatory oligonucleotide	3	1	0
WAVE Life Sciences	Regulatory oligonucleotide	4	0	0
Adhera Therapeutics	Regulatory oligonucleotide	2	1	0
AgonOx	Regulatory oligonucleotide	3	0	0
Amarna Therapeutics	Gene therapy	3	0	0
Ascend Biopharmaceuticals	Gene therapy	2	1	0
Audentes Therapeutics	Regulatory oligonucleotide	3	0	0
Biogazelle	Regulatory oligonucleotide	3	0	0
Bio-Path Holdings	Regulatory oligonucleotide	1	2	0
bluebird bio	Gene therapy	0	1	2
BONAC	Regulatory oligonucleotide	3	0	0
Catalent	Gene therapy	3	0	0
CellGenTech	Gene therapy	3	0	0
Copernicus Therapeutics	Gene therapy	3	0	0
Denovo Biopharma	Gene therapy	1	2	0
Eos Biosciences	Regulatory oligonucleotide	3	0	0
Freeline	Gene therapy	1	2	0
Ibex Biosciences	Gene therapy	3	0	0
Idera Pharmaceuticals	Regulatory oligonucleotide	0	3	0
Medesis Pharma	Regulatory oligonucleotide	3	0	0
MultiVir	Gene therapy	1	2	0
Ocugen	Gene therapy	2	1	0
OncoSec Medical	Gene therapy	1	2	0
PepVax	Gene therapy	3	0	0
Sensorion	Gene therapy	3	0	0

Silenseed	Regulatory oligonucleotide	2	1	0
Vascular Biogenics	Gene therapy	3	0	0
Vivet Therapeutics	Gene therapy	3	0	0
Almirall	Gene therapy	2	0	0
Arrakis Therapeutics	Regulatory oligonucleotide	2	0	0
Aruvant Sciences	Gene therapy	1	1	0
Castle Creek Biosciences	Gene therapy	0	2	0
Cizzle Biotech	Regulatory oligonucleotide	2	0	0
Dicerna Pharmaceuticals	Regulatory oligonucleotide	2	0	0
EdiGene (China)	Regulatory oligonucleotide	2	0	0
Entos Pharmaceuticals	Genome editing	2	0	0
Etubics	Gene therapy	2	0	0
Evotec	Gene therapy	2	0	0
Exonate	Regulatory oligonucleotide	1	1	0
Expansion Therapeutics	Regulatory oligonucleotide	1	1	0
Expression Therapeutics	Gene therapy	1	1	0
Flash Therapeutics	Gene therapy	2	0	0
Genenta Science	Gene therapy	1	1	0
Genprex	Gene therapy	1	1	0
Jazz Pharmaceuticals	Gene therapy	2	0	0
Kolon	Gene therapy	1	1	0
Kubota Pharmaceutical Holdings	Gene therapy	2	0	0
Laboratoires Théa	Regulatory oligonucleotide	2	0	0
Lamellar Biomedical	Regulatory oligonucleotide	2	0	0
Locus Biosciences	Genome editing	0	2	0
miRagen Therapeutics	Regulatory oligonucleotide	3	1	0
Momotaro-Gene	Gene therapy	0	2	0
NanoCarrier	Gene therapy	0	2	0
NanoScope Technologies	Gene therapy	1	1	0
Neuralgene	Gene therapy	2	0	0
OrphageniX	Regulatory oligonucleotide	2	0	0
PharmaMar	Regulatory oligonucleotide	0	2	0
PTC Therapeutics	Gene therapy	1	0	1
Rexahn Pharmaceuticals	Regulatory oligonucleotide	0	2	0
Roivant Sciences	Gene therapy	2	0	0
3-D Matrix	Regulatory oligonucleotide	0	1	0
Advirna	Regulatory oligonucleotide	0	1	0
Allinaire Therapeutics	Regulatory oligonucleotide	1	0	0
Ambulero	Gene therapy	1	0	0
Ambys Medicines	Gene therapy	1	0	0
Amryt Pharma	Gene therapy	1	0	0
Andes Biotechnologies	Regulatory oligonucleotide	0	1	0
AngioGenex	Regulatory oligonucleotide	1	0	0
Antisense Therapeutics	Regulatory oligonucleotide	0	1	0
AnTolRx	Gene therapy	1	0	0
Apeiron Biologics	Regulatory oligonucleotide	0	1	0

Aphios	Regulatory oligonucleotide	1	0	0
Apic Bio	Gene therapy	1	0	0
ArtGen	Gene therapy	1	0	0
ASKA Pharmaceutical	Regulatory oligonucleotide	0	1	0
Bio Sidus	Gene therapy	0	1	0
biOasis Technologies	Gene therapy	1	0	0
Bio-Matrix Scientific Group	Regulatory oligonucleotide	1	0	0
Biomics Biotechnologies	Regulatory oligonucleotide	1	0	0
Cocrystal Pharma	Genome editing	1	0	0
CODA Biotherapeutics	Gene therapy	1	0	0
Cristal Therapeutics	Regulatory oligonucleotide	1	0	0
Cytonus Therapeutics	Genome editing	1	0	0
Defyrus	Gene therapy	1	0	0
Domainex	Regulatory oligonucleotide	1	0	0
Ebelle D'Ebelle Pharmaceuticals	Regulatory oligonucleotide	0	1	0
Elk OrthoBiologics	Gene therapy	0	1	0
Eos Neuroscience	Gene therapy	1	0	0
Errant Gene Therapeutics	Gene therapy	0	1	0
Exicure	Regulatory oligonucleotide	0	1	0
FerGene	Gene therapy	0	1	0
Flexion Therapeutics	Gene therapy	0	1	0
Fondazione Telethon	Gene therapy	0	1	0
Galapagos	Regulatory oligonucleotide	1	0	0
Gene Biotherapeutics	Gene therapy	0	1	0
GeneQuine Biotherapeutics	Gene therapy	1	0	0
Genevant Sciences	Regulatory oligonucleotide	1	0	0
Genovax	Gene therapy	1	0	0
GeoVax Labs	Gene therapy	0	1	0
Gritstone Oncology	Regulatory oligonucleotide	1	0	0
Guangzhou Double Bio-products	Gene therapy	1	0	0
Herantis Pharma	Gene therapy	0	1	0
Highlight Therapeutics	Regulatory oligonucleotide	0	1	0
Holostem Terapie Avanzate	Gene therapy	0	1	0
Horizon Discovery	Gene therapy	1	0	0
Hoth Therapeutics	Regulatory oligonucleotide	1	0	0
Hugel	Regulatory oligonucleotide	0	1	0
Imagene	Gene therapy	1	0	0
Immusoft	Gene therapy	1	0	0
InvivoGen	Gene therapy	0	1	0
Keystone Nano	Regulatory oligonucleotide	1	0	0
Kiromic BioPharma	Gene therapy	1	0	0
Marsala Biotech	Gene therapy	0	1	0
Matinas BioPharma	Regulatory oligonucleotide	1	0	0
Mediphage Bioceuticals	Gene therapy	1	0	0
Medosome Biotec	Gene therapy	1	0	0

Memgen	Gene therapy	0	1	0
miCure Therapeutics	Regulatory oligonucleotide	1	0	0
Morphogen-IX	Gene therapy	2	0	0
Novome Biotechnologies	Genome editing	1	0	0
OcuNexus Therapeutics	Regulatory oligonucleotide	0	1	0
Oisin Biotechnologies	Gene therapy	1	0	0
Omega Therapeutics	Genome editing	1	0	0
Oncotelic	Regulatory oligonucleotide	0	1	0
ORCA Therapeutics	Regulatory oligonucleotide	1	0	0
Oxford BioMedica	Gene therapy	0	1	0
PhageNova Bio	Gene therapy	0	1	0
PharmaPraxis	Regulatory oligonucleotide	1	0	0
PhoreMost	Gene therapy	1	0	0
PNP Therapeutics	Gene therapy	0	1	0
Precision Virologics	Gene therapy	1	0	0
ProCell Therapeutics	Regulatory oligonucleotide	1	0	0
Redbiotec	Genome editing	1	0	0
Reflection Biotechnologies	Gene therapy	1	0	0
Regulus Therapeutics	Regulatory oligonucleotide	1	0	0
Renovacor	Gene therapy	1	0	0
SalioGen Therapeutics	Gene therapy	1	0	0
Santhera Pharmaceuticals	Gene therapy	1	0	0
Scopus BioPharma	Regulatory oligonucleotide	0	1	0
Secarna	Regulatory oligonucleotide	1	0	0
SeleXel	Regulatory oligonucleotide	1	0	0
Shanghai BDgene	Genome editing	0	1	0
Sirtex Medical	Gene therapy	0	1	0
Solasia Pharma	Regulatory oligonucleotide	1	0	0
Solid Biosciences	Gene therapy	0	1	0
SomaGenics	Regulatory oligonucleotide	1	0	0
Stoke Therapeutics	Regulatory oligonucleotide	1	0	0
Synlogic	Gene therapy	1	0	0
Tango Therapeutics	Genome editing	1	0	0
Taysha	Gene therapy	0	1	0
TheraBiologics	Gene therapy	0	1	0
Theragene Pharmaceuticals	Gene therapy	1	0	0
Urovant Sciences	Gene therapy	0	1	0
ValiRx	Gene therapy	1	0	0
Veritas In Silico	Regulatory oligonucleotide	1	0	0
Voyager Therapeutics	Gene therapy	1	0	0
Wings Therapeutics	Regulatory oligonucleotide	0	1	0
XyloCor Therapeutics	Gene therapy	0	1	0