

Pharma R&D Annual Review

Navigating the Landscape



Agenda



Introduction



The drug R&D pipeline in 2022, and how it is changing

- Total pipeline size
- Success stories in 2021
- Top companies
- Leading therapies and diseases
- Mechanisms and targets



Key trends

- COVID, rare diseases, cell and gene therapy, China



Outlook for 2022

- Launches expected from 2021 drug approvals
- Drug approvals to look out for in 2022



Conclusions and Q&A



The total drug R&D pipeline in 2022



Total pipeline size has grown by 8.2%

20,109 drugs under active development

Expansion rate accelerated

- 2021 was up 4.8%
- Above 5-year average of 6.3%

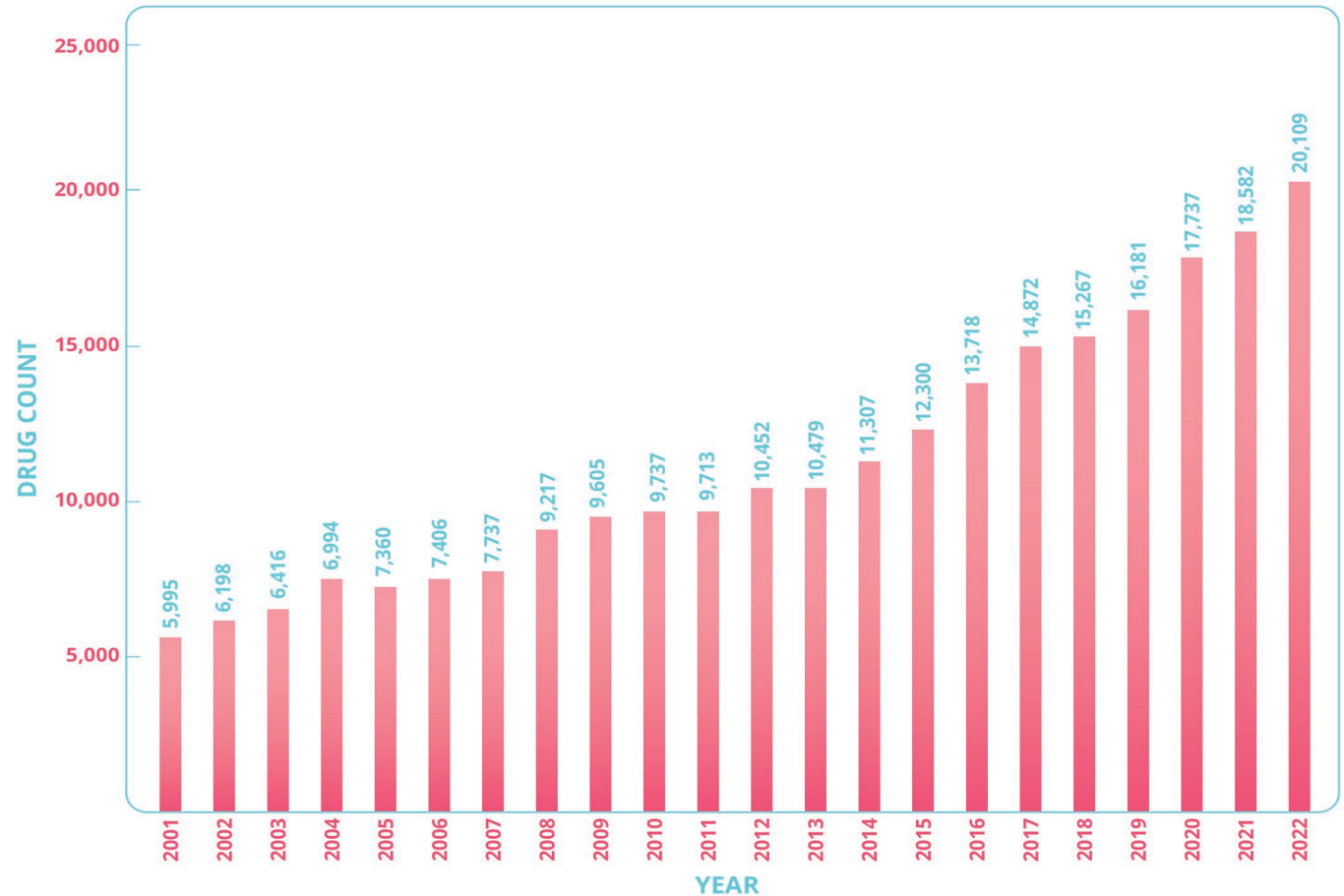
1,527 more drugs in development

- 6,343 new drugs identified during the year
- Vs 5,544 the previous year
- But 4,658 drugs left the pipeline

New drugs statistics

- 38.8% were anticancers
- 14.7% neurologicals
- 12.0% anti-infectives

Total R&D pipeline size, by year, 2001–22



Source: Pharmaprojects®, January 2022

Successes in 2021 – New active substance drug launches



A record year for new drug launches

Pandemic fails to halt new active substance (NAS) roll-outs once again

97 New Active Substances launched

- Across 95 products
- Includes 13 vaccines

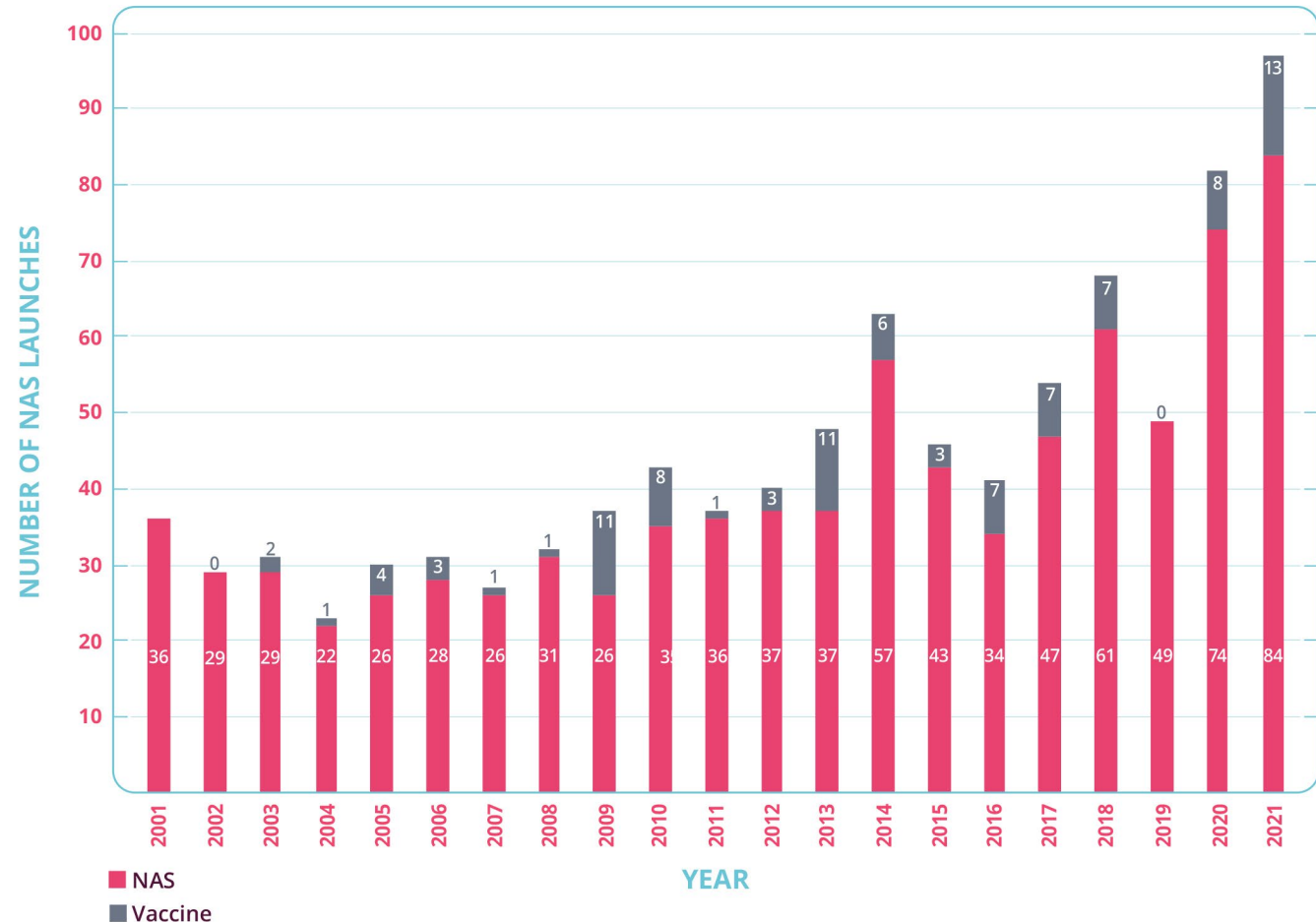
5-year mean hits new heights

- 2017-2021, 63 drugs per year
- Vs 42 in 2012-2016
- > Double seen in the noughties

NAS statistics

- 17 were first in class
- 30 were orphans (31%)
- Cancer most popular
- AstraZeneca had most NAS launches

Number of NAS launches by year, 2001-21



Source: Pharmaprojects®, February 2022

Novel new drug launches 2021

Cancer



Amgen's Lumakras (sotorasib)

- K-Ras inhibitor
- Non-small cell lung cancer
- First launched in the US in August



Merck & Co's Welireg (belzutifan)

- First hypoxia-inducible factor 2 alpha antagonist. HIF-2 α
- For patients with von Hippel-Lindau disease
- First launched in the US in September



Rhizen Pharmaceuticals and TG Therapeutics' Ukoniq (umbralisib)

- PI3 kinase delta inhibitor with the additional, novel pharmacology of casein kinase 1 inhibition
- Marginal zone lymphoma
- First launched in the US in August



Novel new drug launches 2021

Autoimmune/Inflammation



AstraZeneca's Saphnelo (anifrolumab)

- First interferon (type I) receptor antagonist
- Systemic lupus erythematosus
- First launched in Japan in November



RemeGen, part of Rongchang Pharmaceuticals' Tai'ai (telitacicept)

- First APRIL inhibitor
- Fusion protein which jointly targets BLYS
- Launched in China in August for SLE



Hansa Biopharma's Idefirix (imlifidase)

- First drug classified as an endopeptidase stimulant
- Kidney transplant rejection
- Launched in Finland, the Netherlands and Sweden in September



Novel new drug launches 2021

Autoimmune/Inflammation



ChemoCentryx's Tavneos/Vynpenta (avacopan)

- First to target complement C5a receptor 1.
- Anti-neutrophil cytoplasmic autoantibody-associated vasculitis
- First launched in the US in October

Apellis

Apellis' Empaveli (pegcetacoplan)

- First complement C3 convertase inhibitor
- Paroxysmal nocturnal haemoglobinuria
- Launched in the US in July



Novel new drug launches 2021

Dermatology



Leo Pharma's Adbry (tralokinumab)

- First interleukin-13 antagonist
- For atopic eczema
- Launched in the US in December, ahead of rivals



UCB's Bimzelx (bimekizumab)

- A dual interleukin-17A and -17F antagonist
- For use in moderate-to-severe plaque psoriasis
- Debuted in Germany in September

Novel new drug launches 2021

Others



Biogen and Eisai's Aduhelm (aducanumab)

- The first beta-amyloid protein antagonist
- For Alzheimer's disease
- Launched in US in July to much controversy



Regeneron's Evkeeza (evinacumab)

- Angiopoietin-like 3 inhibitor
- For homozygous familial hypercholesterolaemia.
- Launched in US in February



Novel new drug launches 2021

Anti-Infectives



Pfizer's Paxlovid (nirmatrelvir/ritonavir)

- First SARS 3 cysteine-like protease inhibitor
- For COVID-19
- Second direct anti-SARS-CoV-2 antiviral to make it to market



Takeda and GlaxoSmithKline's Livtencity (maribavir)

- First cytomegalovirus UL97 protein kinase inhibitor
- For post-transplant cytomegalovirus infection/disease
- Launched in the US in December



Novel new drug launches 2021

Metabolic



BridgeBio Pharma's Nulibry (fosdenopterin)

- First molybdenum cofactor replacement therapy
- Molybdenum cofactor deficiency
- Launched in the US in May with \$500,000 per year price tag



Eiger BioPharmaceuticals Zokinvy (lonafarnib)

- Hutchinson-Gilford syndrome (progeria) - affects one in 18 million people
- Two novel mechanisms: P-glycoprotein inhibition and MRP inhibition
- Launched in US in January at price of \$1m per year



Lexicon Pharmaceuticals' Zynquista (sotagliflozin)

- Sodium/glucose cotransporter 1 and 2 inhibitor
- First launched for type 1 diabetes in EU and US in December
- It is also awaiting approval for use in type 2 diabetes and heart failure



Other selected new drug launches 2021

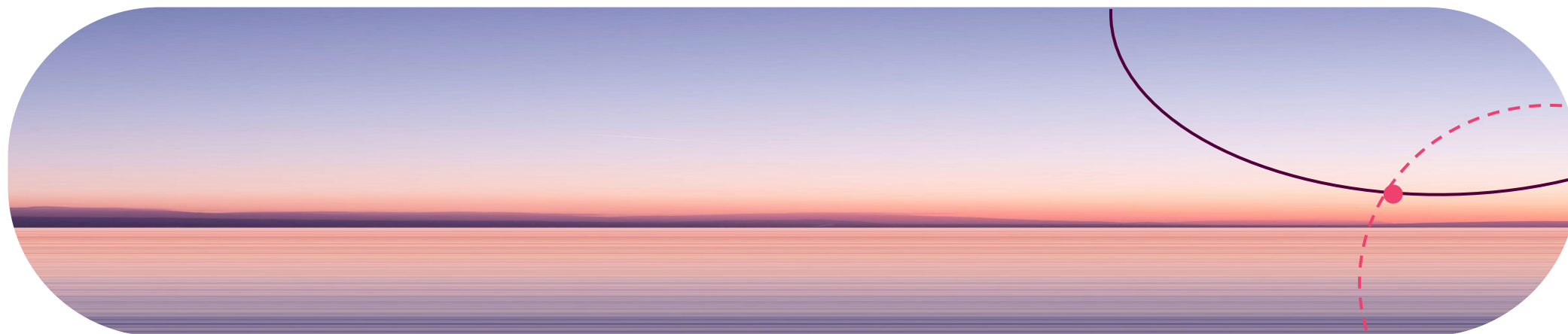
COVID-19 Vaccines

COVID-19 vaccine, Anhui Zhifei Longcom Biopharma	Zifivax	Anhui Zhifei Longcom Biopharma/Changqing Zhifei Biological	COVID-19 prophylaxis
COVID-19 vaccine, AstraZeneca	Covishield	AstraZeneca/Oxford Biomedica	COVID-19 prophylaxis
COVID-19 vaccine, Bharat Biotech-1	Covaxin	Bharat Biotech	COVID-19 prophylaxis
COVID-19 vaccine, CanSino Biologics	Convidecia	CanSino Biologics	COVID-19 prophylaxis
COVID-19 vaccine, CIGB-2	Abdala	CIGB	COVID-19 prophylaxis
COVID-19 vaccine, Finlay Institute-2	Soberana 2	Finlay Institute	COVID-19 prophylaxis
COVID-19 vaccine, Johnson & Johnson	Janssen COVID-19 Vaccine	Johnson & Johnson	COVID-19 prophylaxis
COVID-19 vaccine, Medigen Vaccines Biologics Co.	MVC COVID-19 Vaccine	Dynavax Technologies/Medigen Biotechnology	COVID-19 prophylaxis
COVID-19 vaccine, Shifa Pharmed	COVIran Barakat	Shifa Pharmed	COVID-19 prophylaxis
COVID-19 vaccine, Sinopharm	Sinovac	Wuhan Inst of Biological Sciences/Sinopharm/Chinal National Biotech Group	COVID-19 prophylaxis and treatment
COVID-19 vaccine, Vaxine	SpikoGen	Vaxine/CinnaGen	COVID-19 prophylaxis and treatment

Other selected new drug launches 2021

COVID-19 Treatments

Celltrion's Regkirona (regdanvimab)	COVID-19 prophylaxis and treatment
Eli Lilly's etesevimab	COVID-19 prophylaxis and treatment
Merck & Co/Ridgeback's Lagevrio (molnupiravir)	COVID-19 infection
Pfizer's Paxlovid (nirmatrelvir)	COVID-19 infection
GlaxoSmithKline's Xevudy (sotrovimab)	COVID-19 infection
AstraZeneca's Evusheld (cilgavimab + tixagevimab)	COVID-19 infection and prophylaxis



Other selected new drug launches 2021

CAR-Ts



Bristol Myers Squibb's Abecma (idecabtagene vicleucel)

- For the treatment of relapsed or refractory multiple myeloma
- First launched in the US in May
- Via Celgene acquisition



Bristol Myers Squibb's Breyanzi (lisocabtagene maraleucel)

- For large B-cell lymphoma
- First launched in the US in May
- Via Juno Therapeutics



JW Therapeutics' Relma-cel (relmacabtagene autoleucel)

- For large B-cell lymphoma
- First launched in China in November



Other selected new drug launches 2021

Metabolic Disorders



Albireo Pharma's Bylvay (odevixibat)

- Ileal bile acid transport inhibitor
- For progressive familial intrahepatic cholestasis (PFIC)
- Launched in the US in August



Mirum Pharmaceuticals' Livmarli (maralixibat)

- Ileal bile acid transport inhibitor
- For Alagille syndrome – PFIC indication dropped
- Launched in the US in November



Rhythm Pharmaceuticals' Imcivree (setmelanotide)

- Melanocortin (MC)- 4 receptor agonist and melanocyte-stimulating hormone receptor agonist
- For POMC deficiency/Leptin receptor deficiency obesity
- Launched in the US in September



Other selected new drug launches 2021

Additional therapeutic areas



Novartis Leqvio (inclisiran)

- Heterozygous familial hypercholesterolemia
- RNA interference product obtained via The Medicines Company
- Launched in the EU in November



Sarepta Therapeutics' Amondys 45 (casimersen)

- Third exon-skipping splice switching oligomer
- For Duchenne's muscular dystrophy
- Launched in the US in June following accelerated approval



Bayer and Merck & Co's Verquvo (vericiguat)

- Guanylate cyclase stimulant
- For use in heart failure patients
- Launched in Japan in September





Most successful companies for drug launches in 2021

AstraZeneca leads the charge

AZ launches five NASs

- COVID-19 vaccine and dual Mab therapy included

Four firms launch NAS trios

- Of these Bayer leads in NAS launch to pipeline size ratio
- Roche missing from top 10

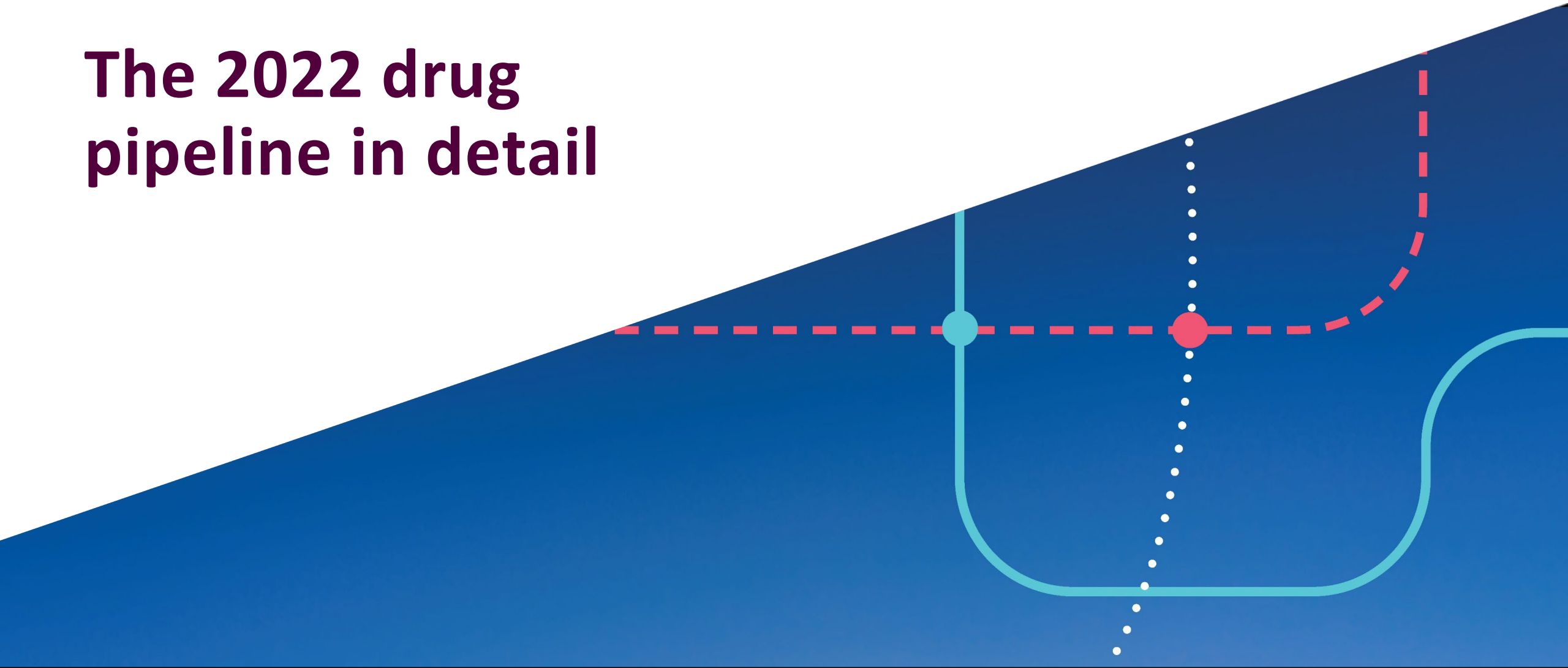
Chinese firms make mark

- Four launch two apiece
- Guangzhou Pharmaceuticals has best NAS:size ratio

Top company NAS launch performance, 2021

COMPANY	NO. OF NAS LAUNCHES 2021	POSITION BY PIPELINE SIZE
AstraZeneca	5	6
Bayer	3	14
GlaxoSmithKline	3	11
Johnson & Johnson	3	8
Merck & Co	3	7
Guangzhou Pharmaceuticals	2	4,149
RemeGen/Rongchang Pharmaceuticals	2	457
Ascentage	2	336
Hutchmed	2	212
BridgeBio Pharma	2	100
Innovent Biologics	2	54
Sumitomo Dainippon	2	25
Eisai	2	18
Jiangsu Hengrui Pharmaceuticals	2	16
Pfizer	2	5
Bristol Myers Squibb	2	4
Takeda	2	3
Novartis	2	1
Eli Lilly	1	10
Sanofi	1	9
Roche	0	2

The 2022 drug pipeline in detail





Breakdown of the pipeline by phase

More drugs at each development stage

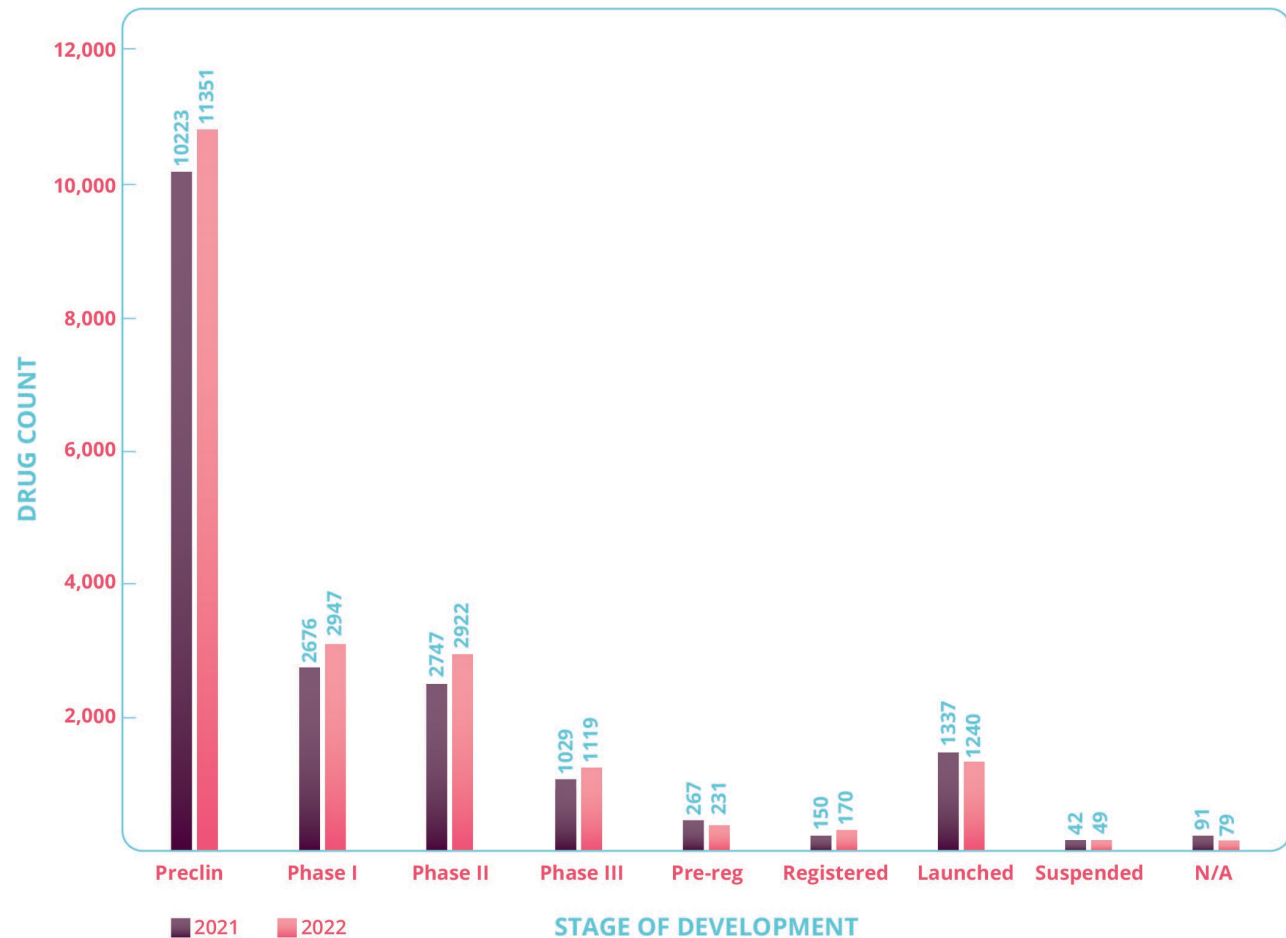
Biggest increases at early stages

- Preclinical up by 11.0%
- Phase I up by 10.1%
- 6,343 drugs added during 2021

Later clinical stages also rise

- Phase II up by 6.4%
- Phase III up by 8.7%

Pipeline by development phase, 2022 versus 2021

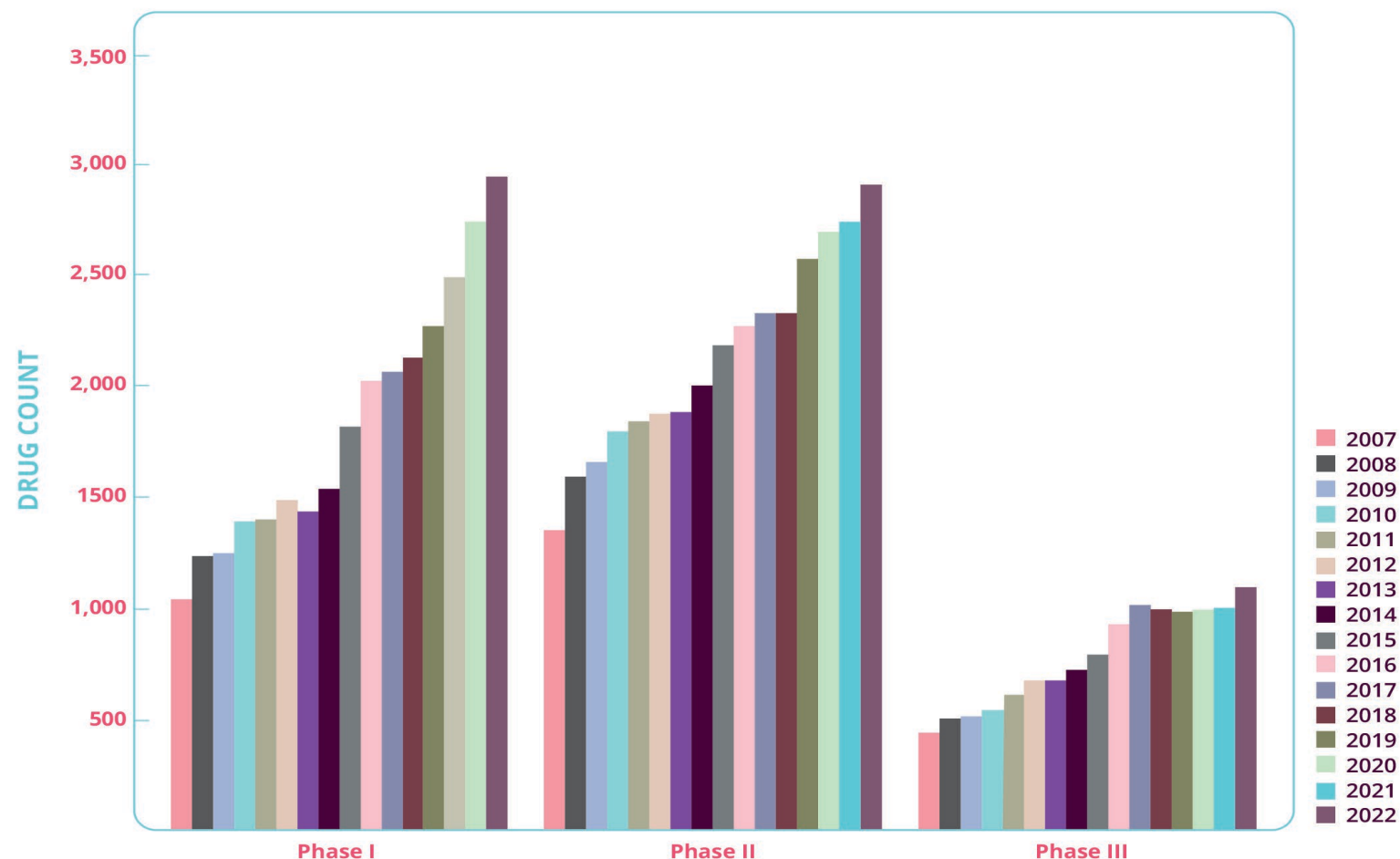


N/A: not applicable and is applied to companion diagnostics prelaunch

Trends in drug R&D by clinical phase

Phase III numbers on the rise again after years of stagnation

Clinical phase trends, 2007–22



Top 10 pharma companies by pipeline size

Little change in the Top 10

Novartis spends 6th year at the top

- Top 4 unchanged
- Lilly re-enters Top 10 at the expense of AbbVie

Most pipelines shrink

- Only AZ, Sanofi and Lilly grow
- AZ acquired Alexion
- But overall, another quiet year for M&A amongst the Top 10

Position 2022 (2021)	Company	No of Drugs in Pipeline 2022 (2021)	No of Originated Drugs 2022	Trend
1 (1)	Novartis	213 (232)	129	↓
2 (2)	Roche	200 (227)	120	↓
3 (3)	Takeda	184 (199)	68	↓
4 (4)	Bristol-Myers Squibb	168 (177)	98	↔
5 (6)	Pfizer	168 (170)	101	↔
6 (9)	AstraZeneca	161 (157)	89	↔
7 (5)	Merck & Co	158 (176)	77	↓
8 (7)	Johnson & Johnson	157 (162)	86	↔
9 (10)	Sanofi	151 (141)	87	↔
10 (11)	Eli Lilly	142 (126)	76	↑

Big pharma providing a diminishing slice of the pipeline

Mid-tier companies fuel growth

Top 10 provides 4.6% of all R&D

- Down from 5.3%
- Rate of decline increased somewhat

Top 25 down to 8.5%

- Less than half the % of 10 years ago

% from minnows remains around 17%

- Fall despite a similar number of companies
- Mid-size companies fuelling pipeline growth

Share of the pipeline contributed by top 10 companies, top 25 companies, and companies with just one or two drugs, 2011–22



Disease focus areas of the Top 10 pharma companies

Big pharma active in all Therapeutic Areas

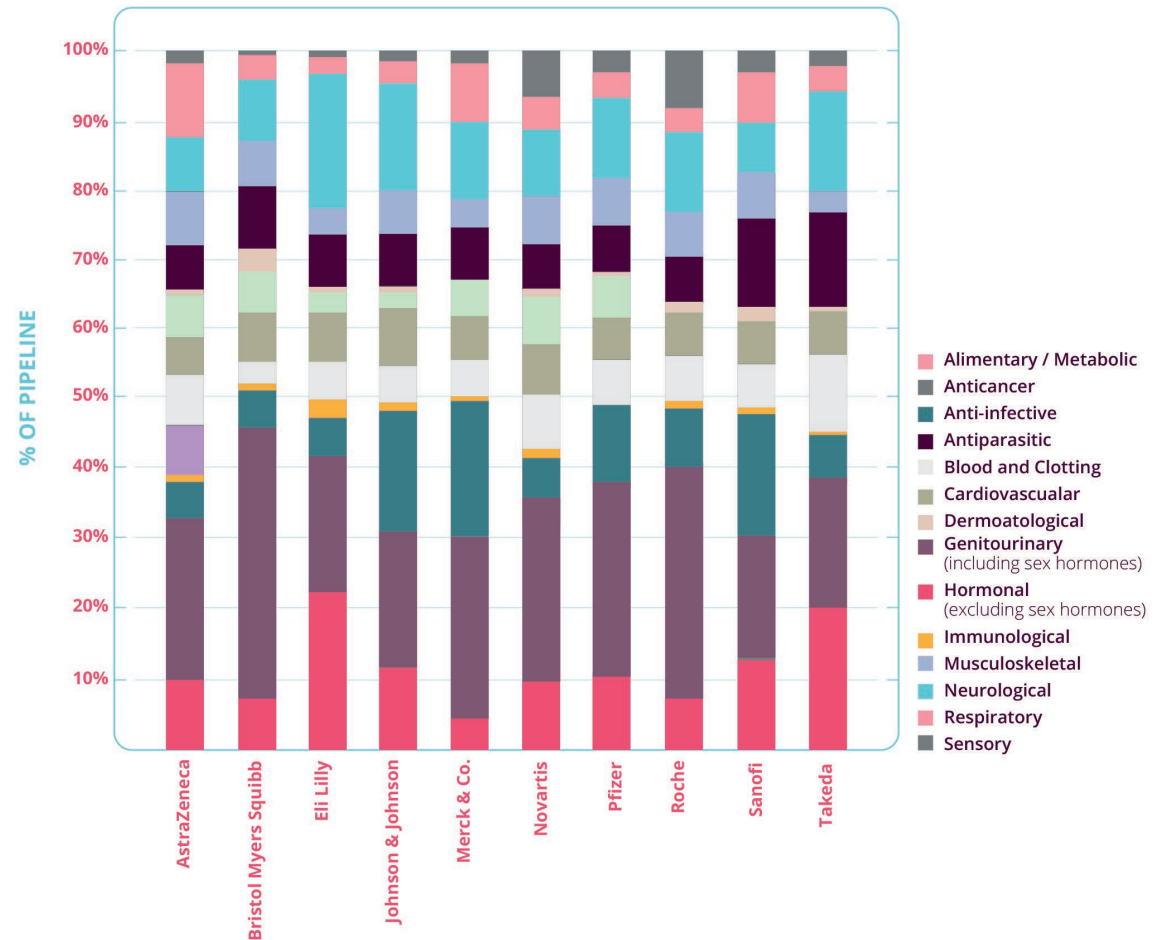
Cancer major focus for all

- BMS has biggest focus on cancer
- Leading TA for all except Eli Lilly

Big pharma still favours broad portfolios

- 8/10 active in all 14 TAs
- Merck & Co has largest % anti-infective pipeline

Disease focus areas of the top 10 pharma companies



Total number of companies with active pipelines 2001-2022

Pharma's world expands by another 6.2%

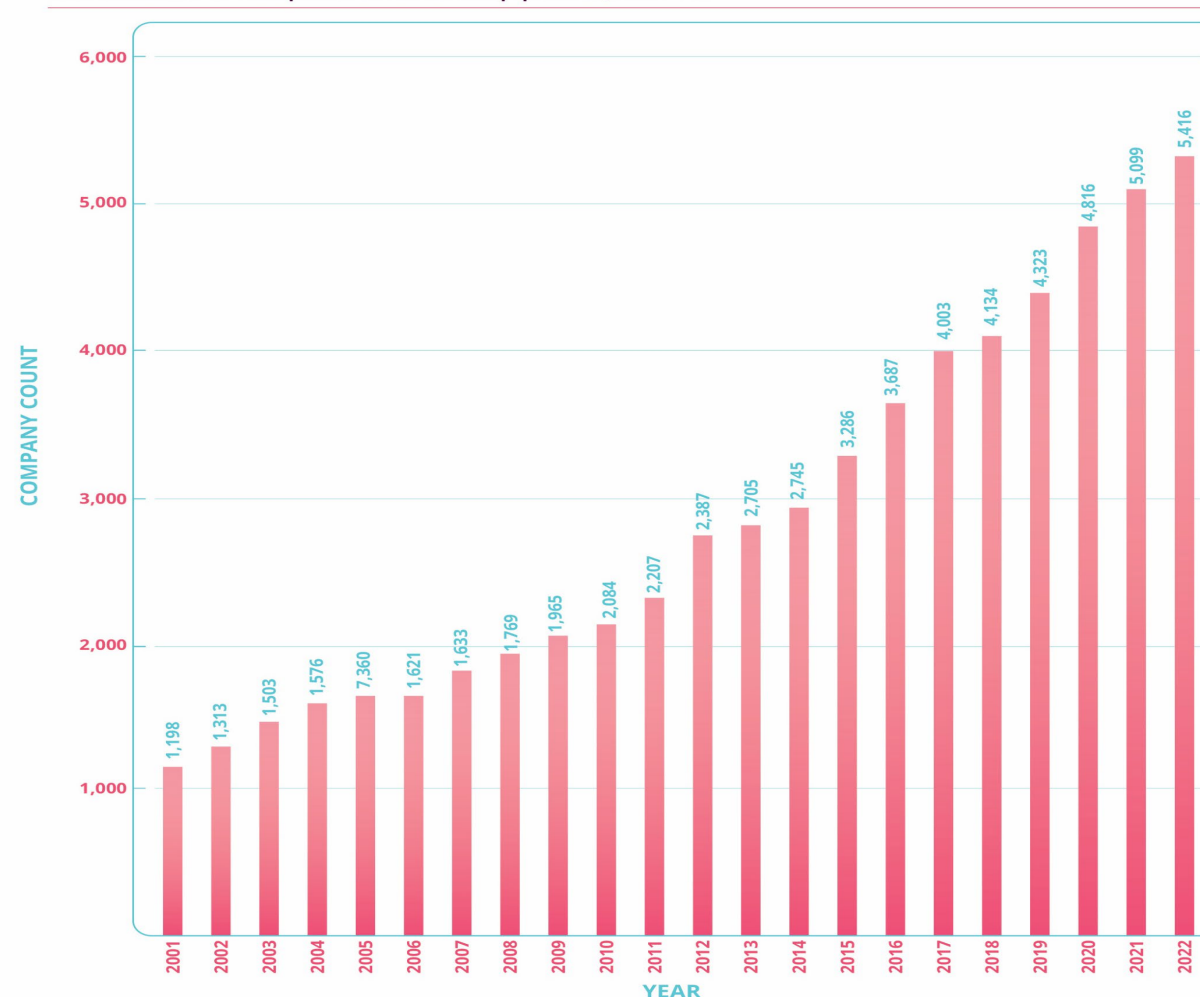
Over 1,000 new companies

- 1,042 firms join pharma family
- Net of 725 left
- 19.2% of companies emerged in past year

Over half are tiny

- 759 companies with 2 drugs
- 1,883 with just a single project

Total number of companies with active pipelines, 2001–22



The R&D pipeline by Therapeutic Area

Cancer stays top

Cancer ever more dominant

- 39.0% of all drugs have an oncology target
- Up from 37.5% one year go; 29.5% ten years ago
- Grew by 14% this year

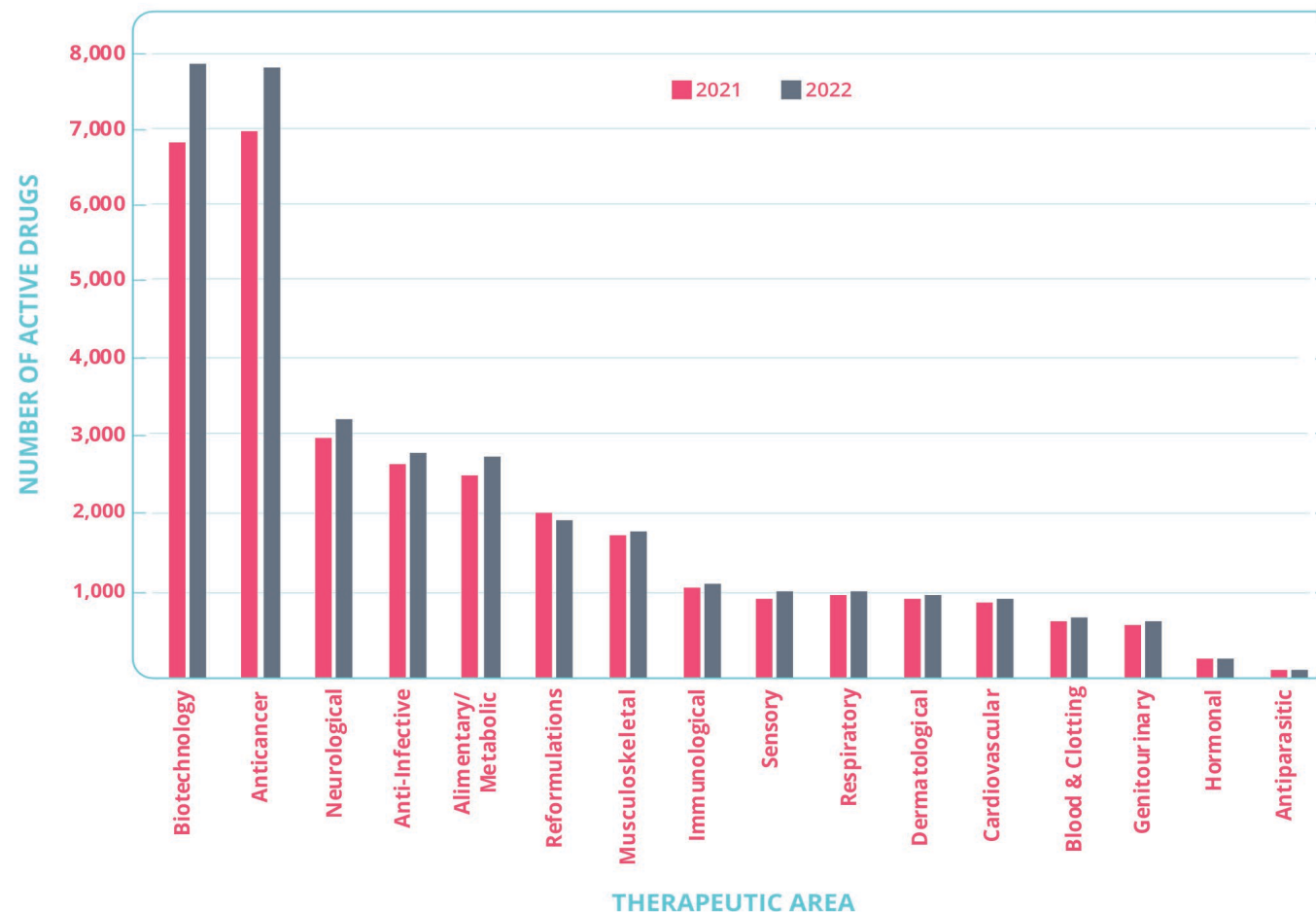
Neurologicals in second

- Grew by 7.8%

Anti-infectives third

- Up by 4.7%
- Much slow growth rate than 22.4% seen in 2021

The R&D pipeline by therapy group, 2021 and 2022



Top 10 diseases/indications

Cancer still takes 7 of the Top 10

Breast cancer top target

- But growth stalls

NSCLC catching up

- 11.5% pipeline expansion

COVID still expanding

- Up by 22.4%

Alzheimer's rallies

- Aduhelm effect?

Position 2022 (2021)	Disease	No of Active Drugs 2022 (2021)	Trend
1 (1)	Cancer, breast	888 (886)	↔
2 (2)	Cancer, lung, non-small cell	832 (746)	↑↑
3 (5)	Infection, coronavirus, novel coronavirus	677 (553)	↑↑
4 (3)	Cancer, colorectal	663 (653)	↔
5 (4)	Cancer, pancreatic	591 (570)	↔
6 (6)	Cancer, ovarian	530 (518)	↔
7 (7)	Cancer, prostate	509 (481)	↔
8 (9)	Alzheimer's disease	496 (436)	↑
9 (8)	Cancer, brain	485 (460)	↔
10 (11)	Cancer, leukaemia, acute myelogenous	462 (430)	↑



Top 10 mechanisms of action (pharmacologies)

Immuno-oncology and associated mechanisms dominate

IO up a further 14.8%

- But only 2% late-stage

Other IO-related rises

- Checkpoint inhibitors up by a third
- CD3 nearly doubles

Genome editing and gene expression inhibition advance

New entry for protein degraders

Position 2022 (2021)	Mechanism of Action	No of Drugs 2022 (2021)	% at PR, R or L	Trend
1 (1)	Immuno-oncology therapy	3,307 (2,880)	2.1	↑
2 (2)	Immunostimulant	1,494 (1,410)	8.6	↑
3 (3)	T cell stimulant	1,062 (906)	1.0	↑
4 (4)	Immune checkpoint inhibitor	575 (432)	4.9	↑↑
5 (9)	Genome editing	280 (173)	0	↑↑
6 (5)	Gene expression inhibitor	262 (191)	1.9	↑
7 (6)	Angiogenesis inhibitor	198 (190)	26.3	↔
8 (-)	Protein degrader	197 (-)	1.0	↑↑
9 (17)	CD3 agonist	196 (100)	2.0	↑↑
10 (8)	Radiopharmaceutical	183 (174)	9.3	↔

Top 10 drug protein targets

Cancer and, in particular, IO targets dominate

CD3e now most popular target

- Key target for bispecific antibodies

Other IO-related rises

- PD-L1 up to No.2
- CD19, PD-1 also up

Top 7 all cancer targets

Boost for 5-HT2A

- 43 new drugs identified in 2021

Position 2022 (2021)	Target	No of Active Drugs 2022 (2021)	Trend
1 (3)	CD3e molecule	199 (149)	↑↑
2 (6)	CD274 molecule [<i>PD-L1</i>]	194 (141)	↑↑
3 (1)	erb-b2 receptor tyrosine kinase 2 [<i>Her-2</i>]	177 (16)	↔
4 (4)	CD19 molecule	174 (144)	↑
5 (2)	epidermal growth factor receptor	161 (151)	↔
6 (7)	programmed cell death 1 [<i>PD-1</i>]	159 (122)	↑
7 (5)	vascular endothelial growth receptor factor A	158 (142)	↔
8 (10)	glucagon like peptide 1 receptor	116 (98)	↑
9 (8)	opioid receptor mu 1	104 (112)	↓
10 (23)	5-hydroxytryptamine receptor 2A	103 (60)	↑↑

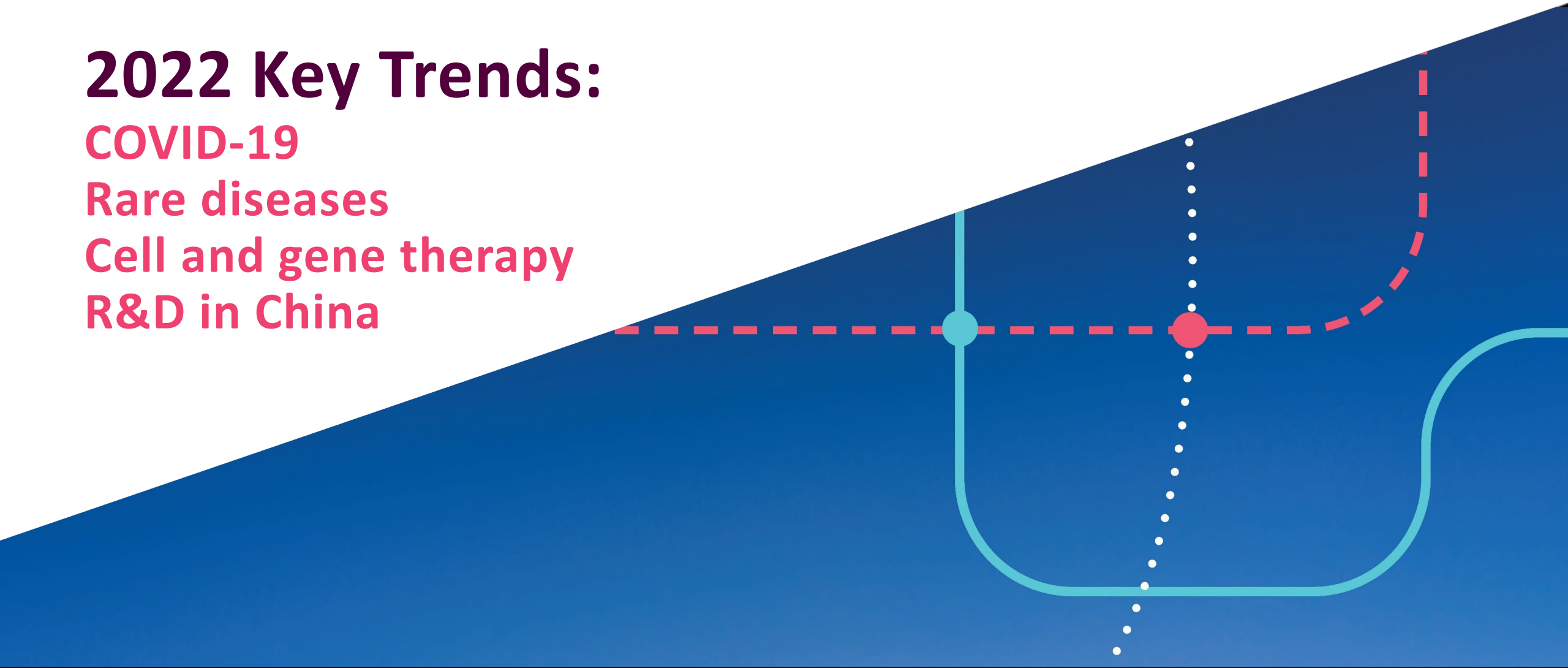
2022 Key Trends:

COVID-19

Rare diseases

Cell and gene therapy

R&D in China





Response to COVID-19 from pharma continued

New candidates peaked in April 2020

Second year sees fewer new candidates

- New complication therapies fall back
- New vaccine R&D sustained

Will 2022 see further declines?

- Focus likely to switch to antivirals

COVID-19 response in years one and two of the pandemic



COVID-19 response from Top 10 pharma companies

All the Top 10 playing their part

Pfizer has the biggest presence

- Leads on vaccines

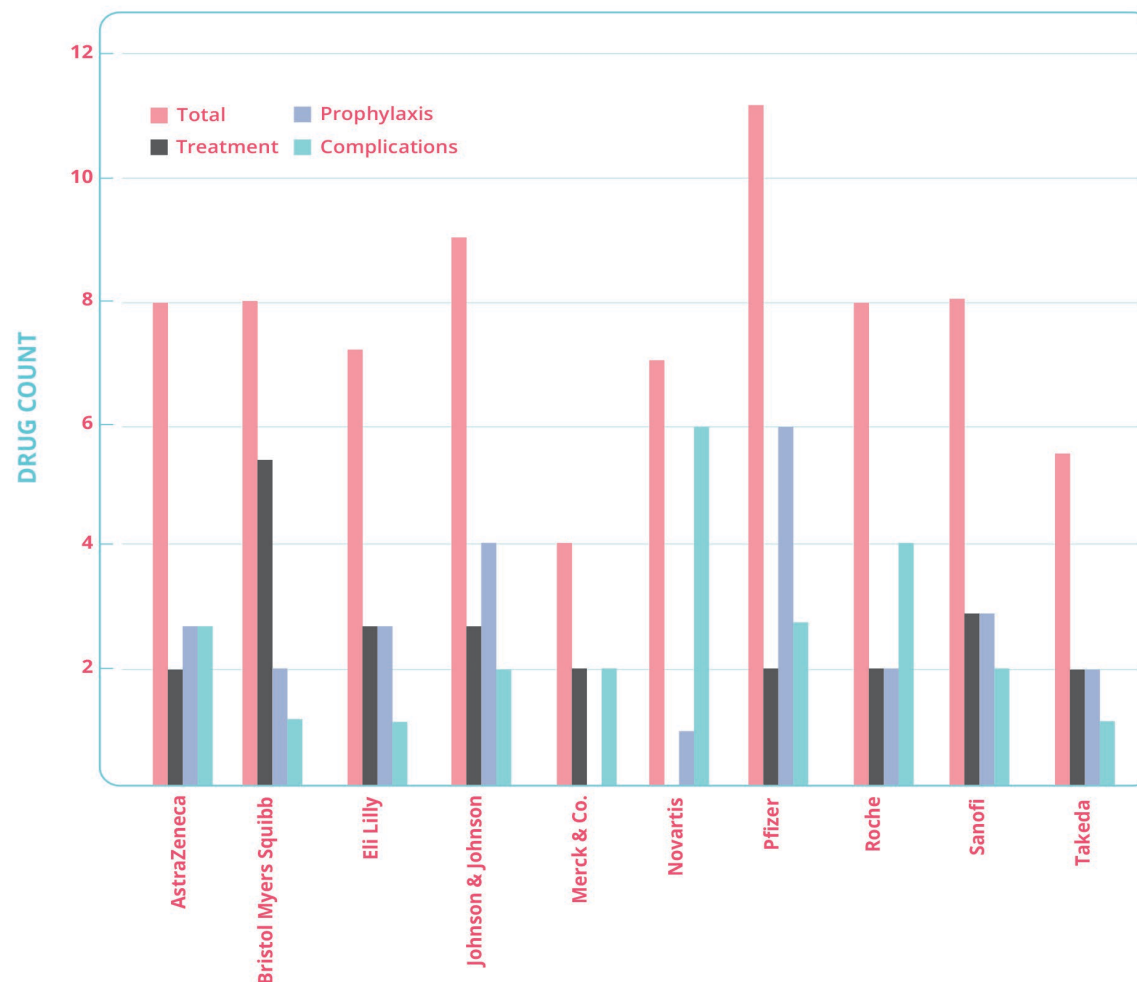
BMS leads on antivirals

Roche has most complication therapies

Merck has smallest pipeline

- Avoids vaccines entirely

COVID-19 pipelines at the top 10 pharma companies





Rise of R&D for rare diseases

Number of rare diseases targeted hits 677

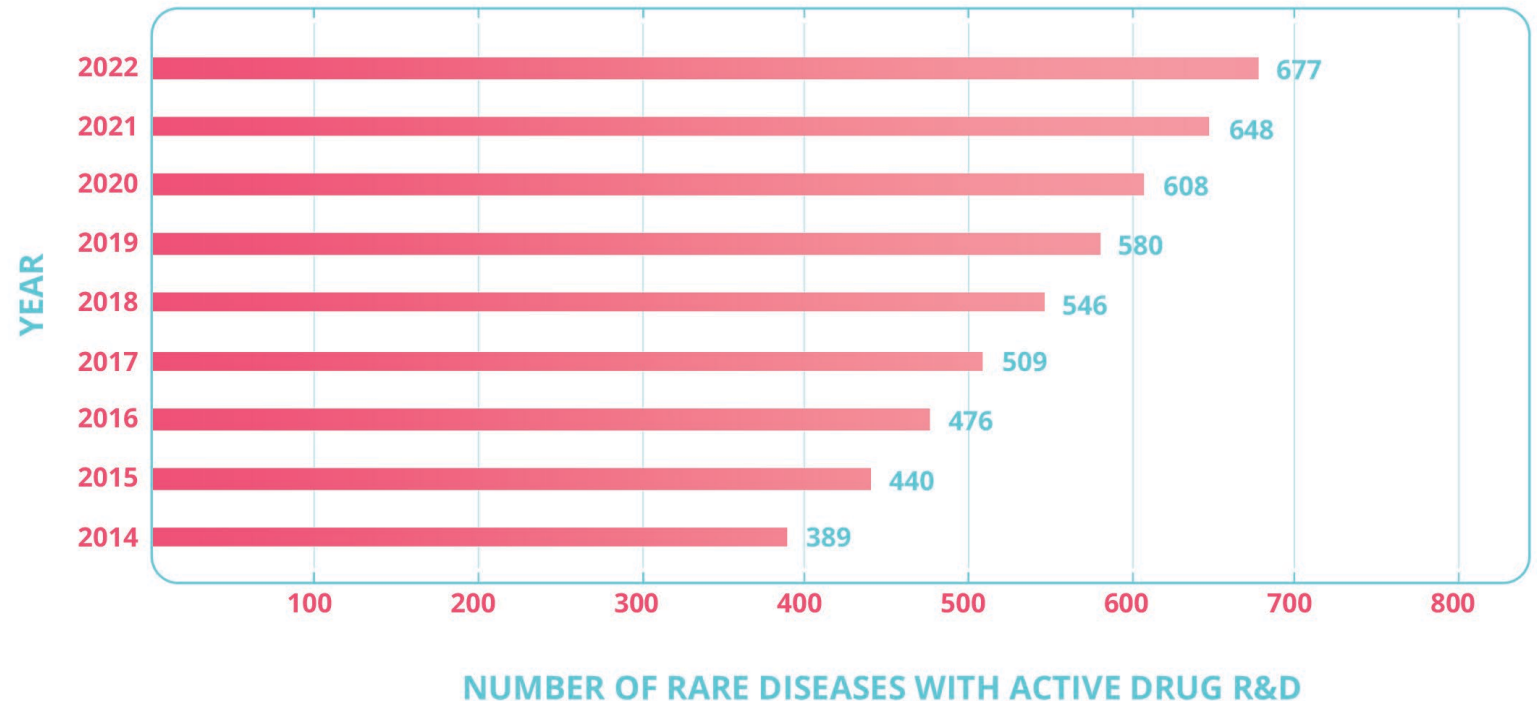
Up from 648 last year

- Almost double that covered in 2014

Number of drugs up too

- 6,080 drugs in development for at least one rare disease
- Up from 5,608 last year
- Represents 30.2% of the pipeline

Number of rare diseases being targeted by pharma, 2014-22



Therapeutic Area focus of rare disease R&D

Good spread across the TAs

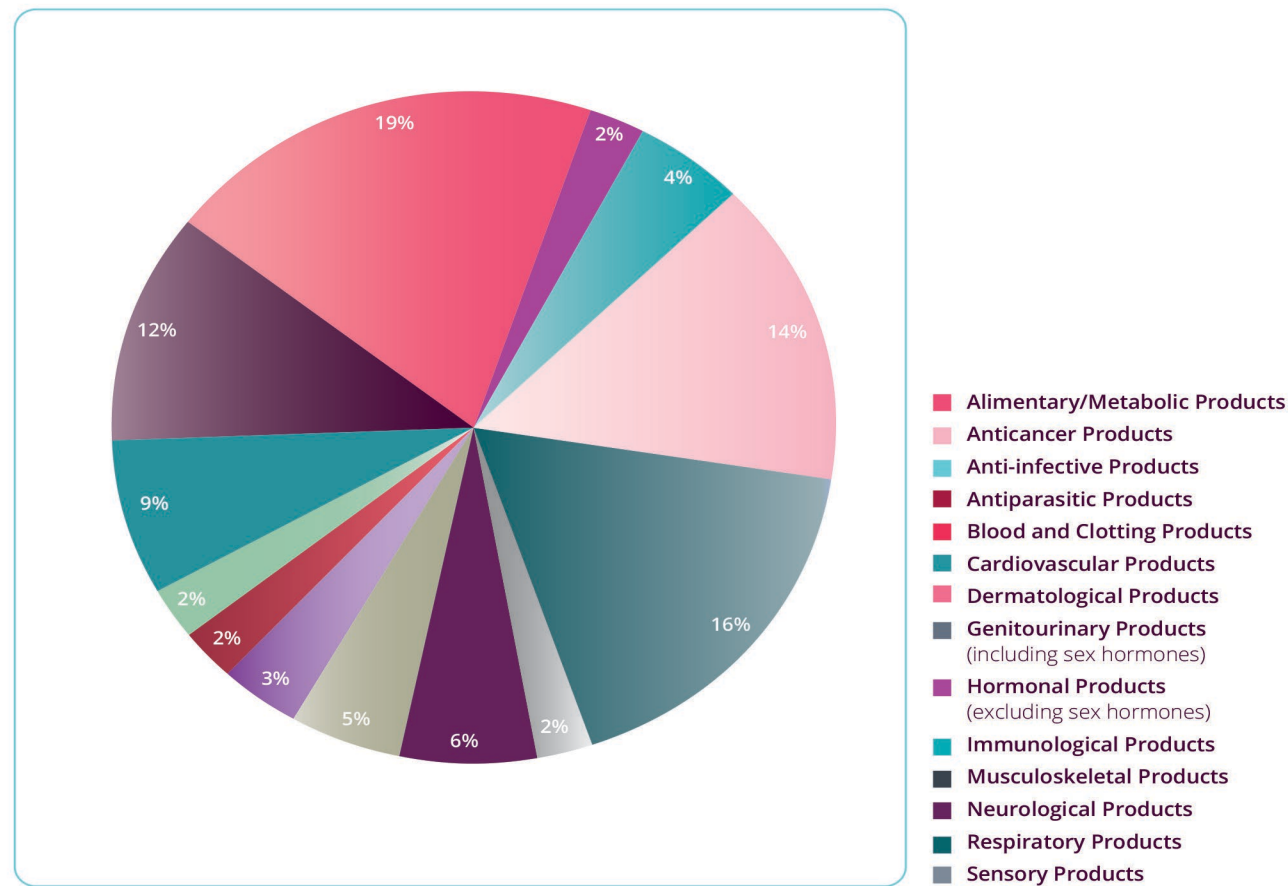
Alimentary/Metabolic has largest share

- Includes many single gene defect metabolic and enzyme disorders

Neurologicals second

- Cancer third with 14%
- Infectious diseases on 12%

Rare diseases by therapeutic area





Rare diseases a big focus for Big Pharma

All are in the rare disease Top 20

Novartis tops this chart too

- 138 drugs for rare diseases
- 64.8% of its pipeline

Big pharma dominates

- 9 of the Top 11 are Top 10 pharma companies
- Eli Lilly has smallest %

Niche company features too

- Shape Therapeutics has largest % in the Top 20

Top 20 pharma companies with a rare disease focus

COMPANY	NO. OF DRUGS FOR RARE DISEASES	% OF PIPELINE
Novartis	138	64.8
Bristol Myers Squibb	108	64.3
Pfizer	98	58.3
Roche	96	48.0
Sanofi	96	63.6
Takeda	92	50.0
AstraZeneca	78	48.4
Johnson & Johnson	69	43.9
GlaxoSmithKline	68	51.9
AbbVie	59	48.8
Merck & Co	55	34.8
Amgen	54	65.1
Bayer	49	46.7
Biogen	40	60.6
Eli Lilly	40	28.2
Eisai	38	47.5
Otsuka Holdings	38	40.9
Shape Therapeutics	37	69.8
BeiGene	36	55.4
Astellas Pharma	34	45.3

NOTE: A rare disease is defined as one with a prevalence of 1 in 2,000 people in the EU, or affecting fewer than 200,000 people in the US (equivalent to around 1 in 1,600 people).

Clinical trial starts in rare disease R&D

Record broken in 2021

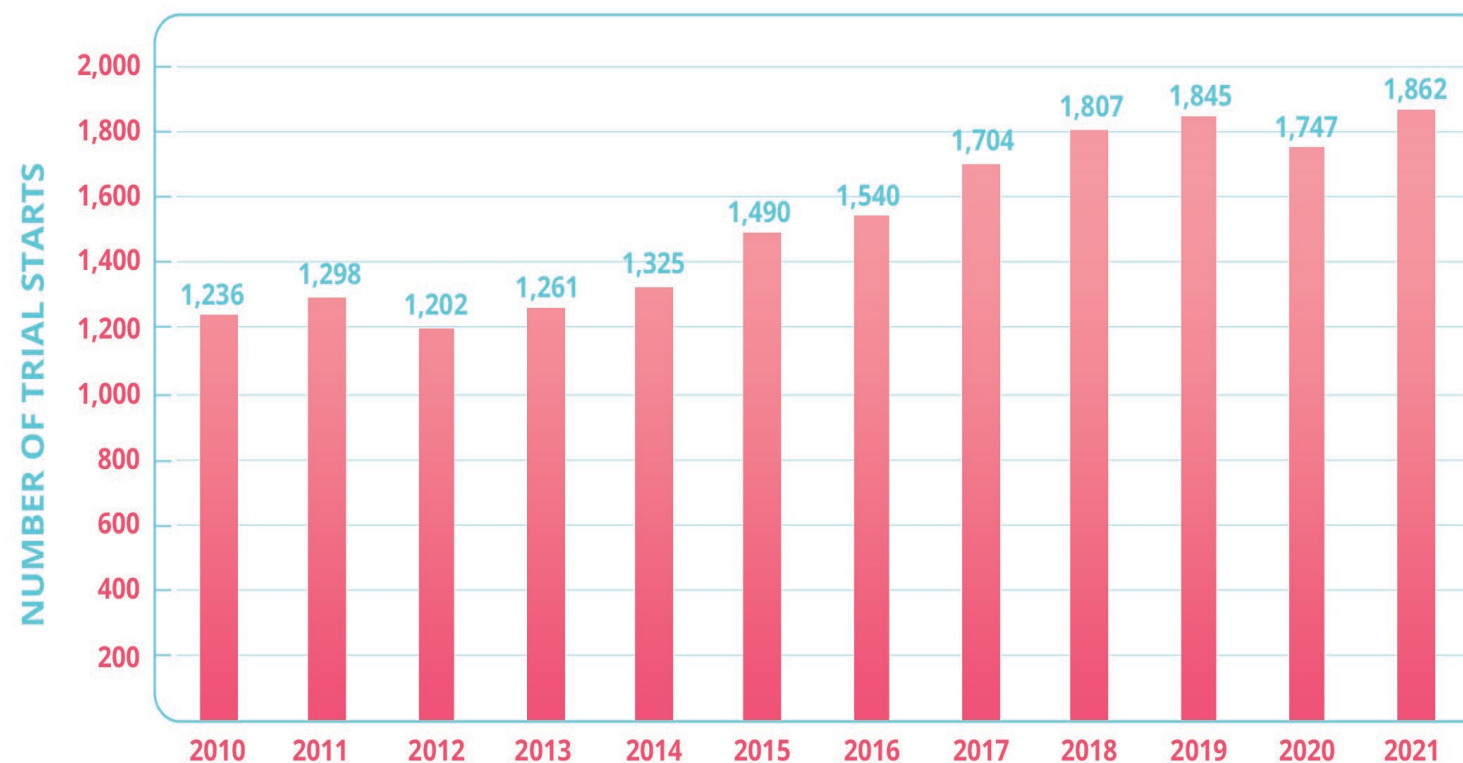
2021 saw most rare disease trial starts

- Followed slight dip in 2020
- Reporting delay means this figure could rise further

Overall, Trialtrove reports that oncology trials dominate

- Over 16,000 ongoing oncology trials
- Dwarfs CNS in 2nd with 6,000
- Over 6,500 trials in COVID since the pandemic's start

Industry-sponsored rare disease trials by start date, 2010-21



ODS, ERD and EUAs on the rise

Keeping pace with rare disease R&D

381 drugs granted Orphan Drug Status

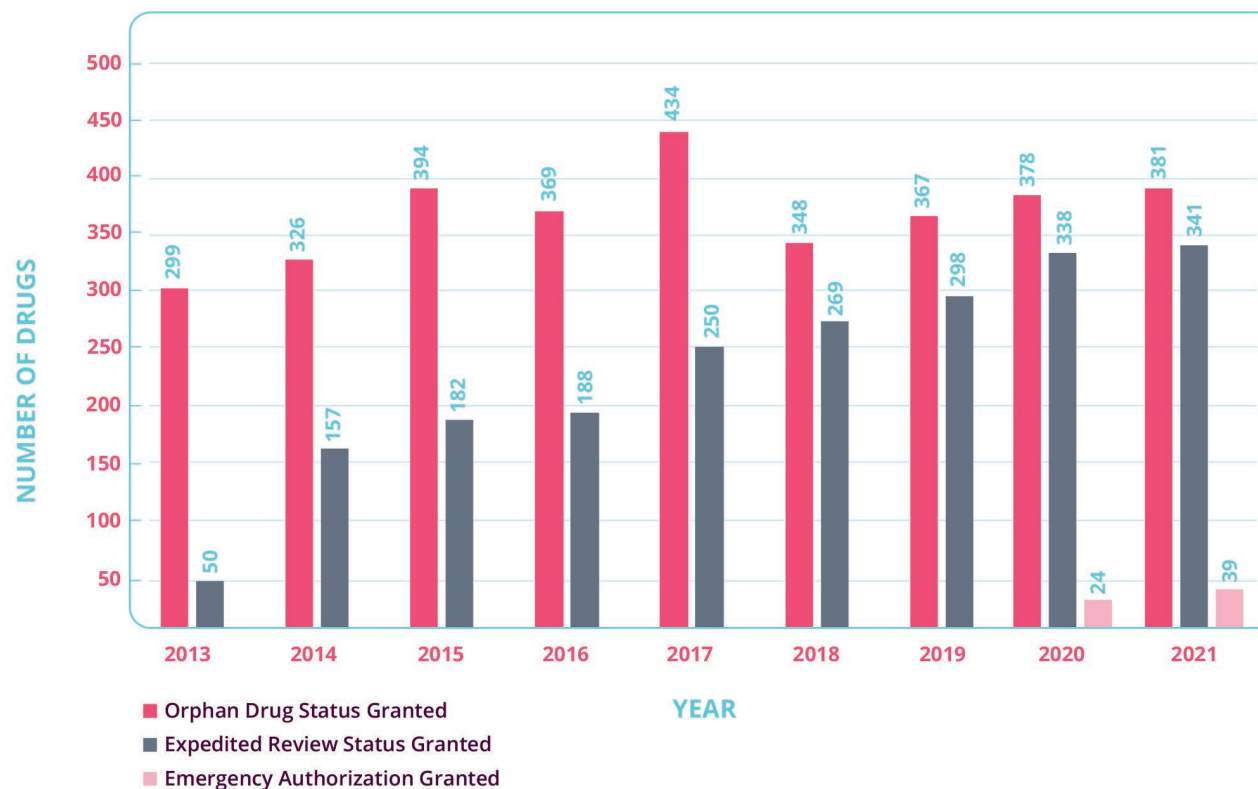
- 3rd largest on record

341 Expedited Review Designations granted

- Most ever in a single year

39 Emergency Use Authorizations granted

Number of drugs receiving Orphan Drug status, Expedited Review designation*, and Emergency Authorization**, 2013-21



Data for 2013 not complete as we only began systematically recording the dates of these events mid-year.
Emergency Authorizations only tracked from 2019.

Cell and gene therapies soaring

Gene therapy regains the lead

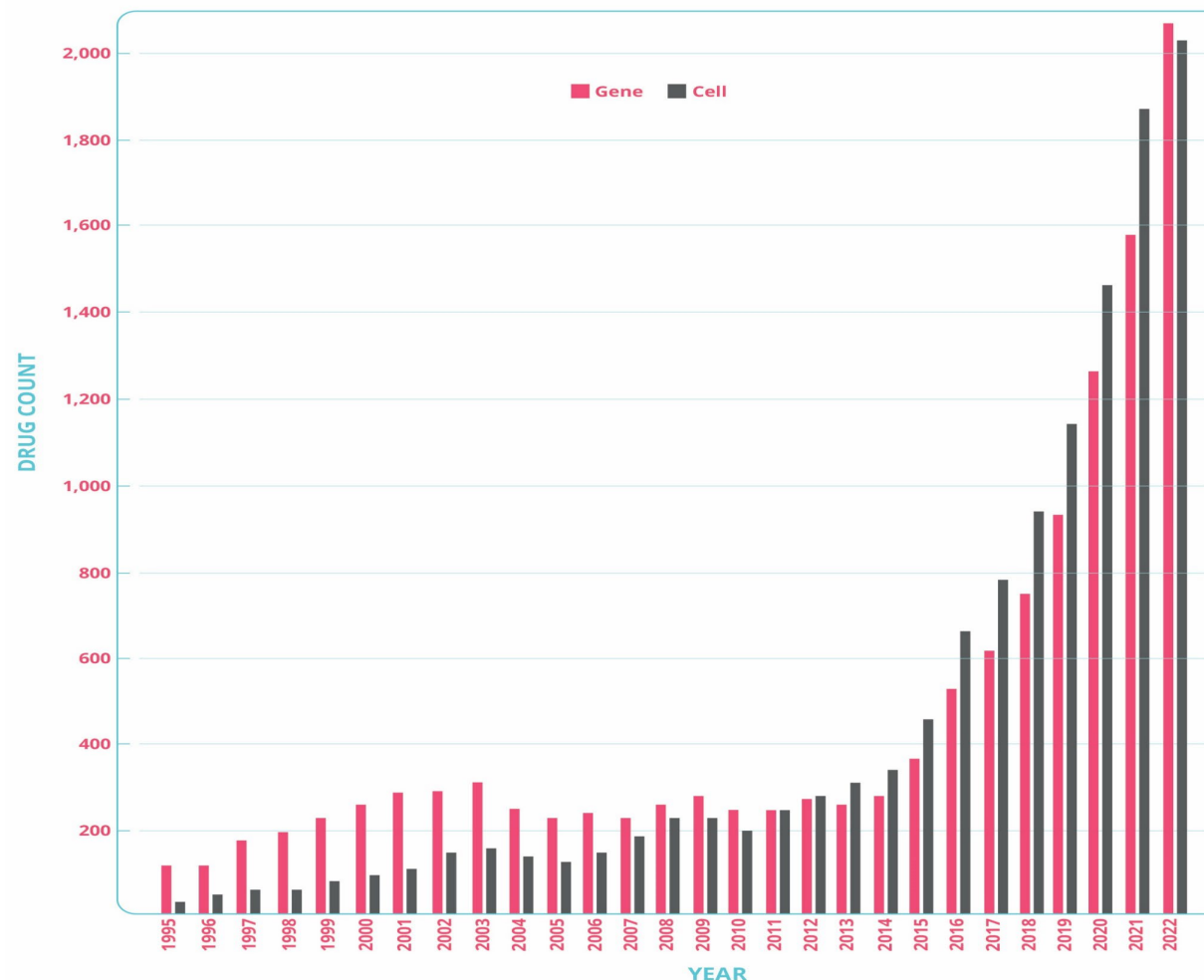
Gene therapy tops 2K

- Includes in vivo and ex vivo (eg CAR-T)
- Recovery after early '00s setbacks
- 17 on market, including 6 CAR-Ts
- Recent regulatory difficulties could put the breaks on

Cell therapies also top 2K

- 57% also have a gene therapy component

The ongoing rise of gene and cell therapy



Viral vectors used in gene therapy

AAV most popular vector

Boom in use of adeno-associated virus

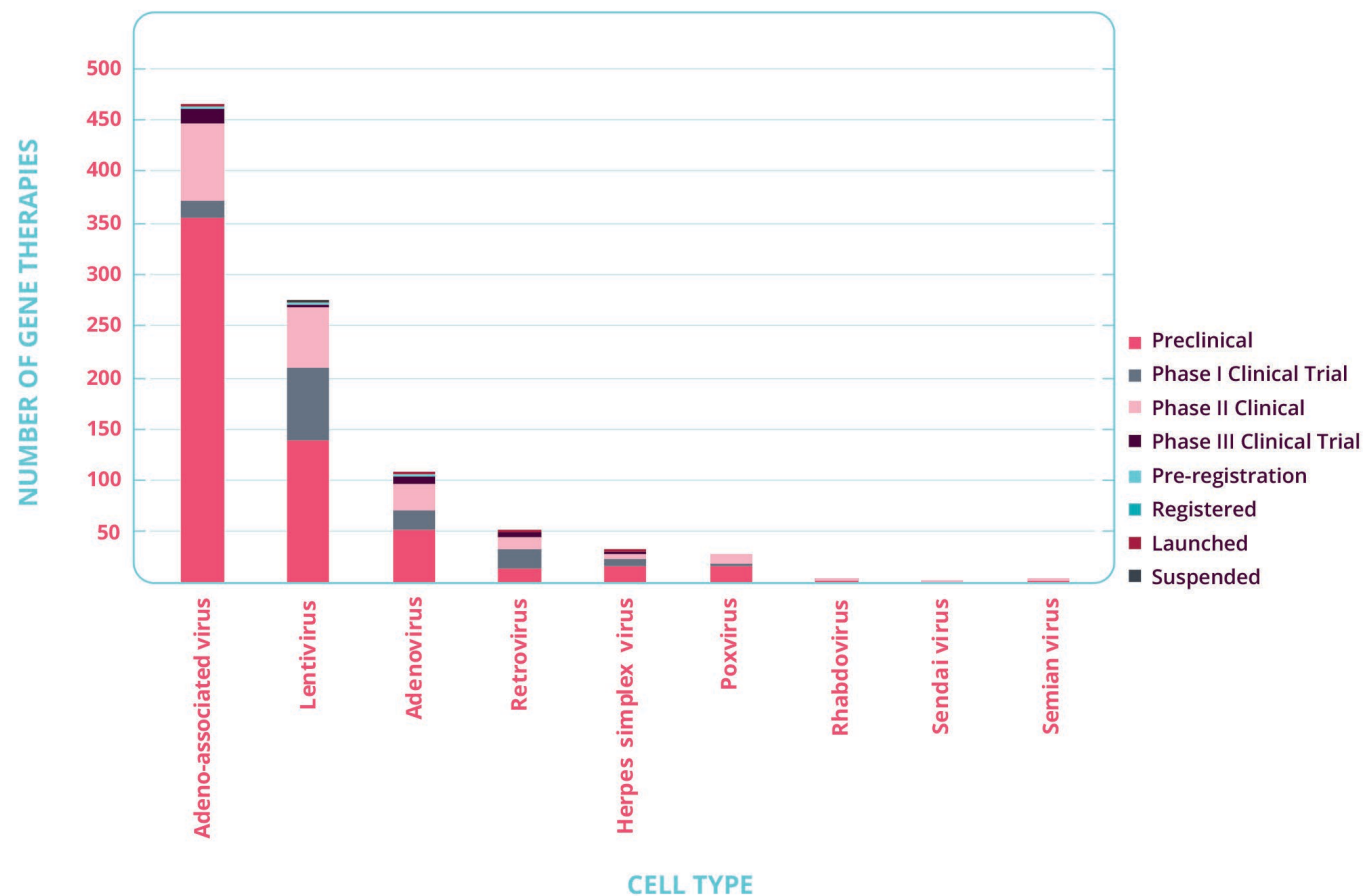
- 466 use this vector
- Up from 341 in 2021

Lentivirus claims 2nd place

- Used in ex vivo gene therapy to transfect cells

Retroviral vectors also showed robust growth

Viral vectors used in gene therapy



Cell types used in cell therapy

T cells are top choice

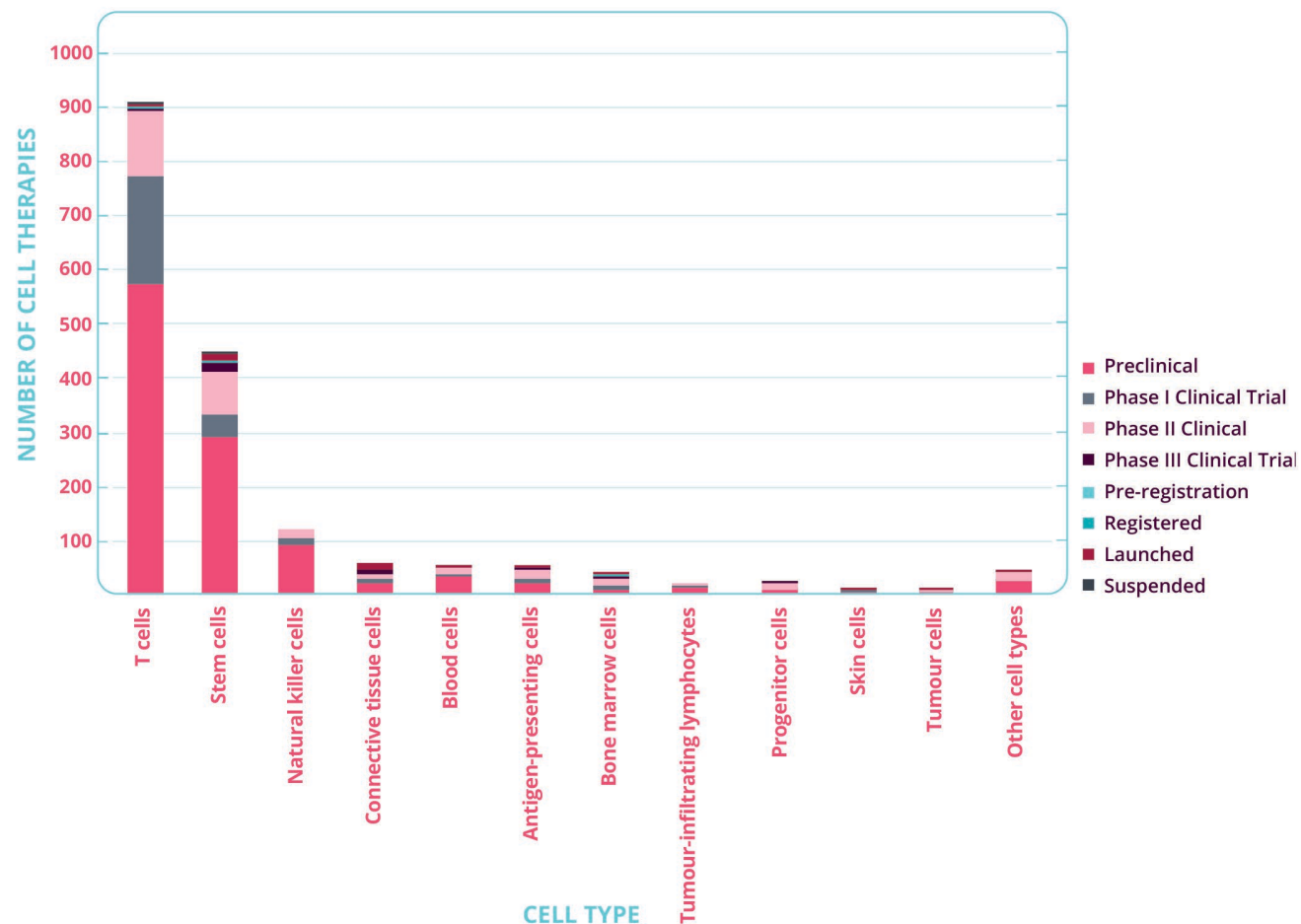
Over 900 T cell-based therapeutics in development

- Includes over 700 CAR-Ts

Stem cells next most popular

- Around 450 projects
- NK cells come in third

Cell types used in cell therapies



Landmark year for Chinese pharma R&D

Phenomenal growth takes China into the big league



Chinese companies in the Top 25 companies by pipeline size for the first time

- Jiangsu Hengrui at No. 16, up from 37, with a 71% increase in pipeline size
- Shanghai Fosun at No. 23, up from 66, with a 127% increase in pipeline size
- BeiGene just outside the Top 25

China the first market for 19% of New Active Substances (NASs)

- Up from 8.5% in 2020
- Three Chinese companies delivered >1 NAS (Jiangsu Hengrui, Guangzhou Pharma, RemeGen/Rongchang)
- China-originated NASs starting to make an impact



Chinese HQed companies up nearly 50%

Now account for 12% of all R&D firms

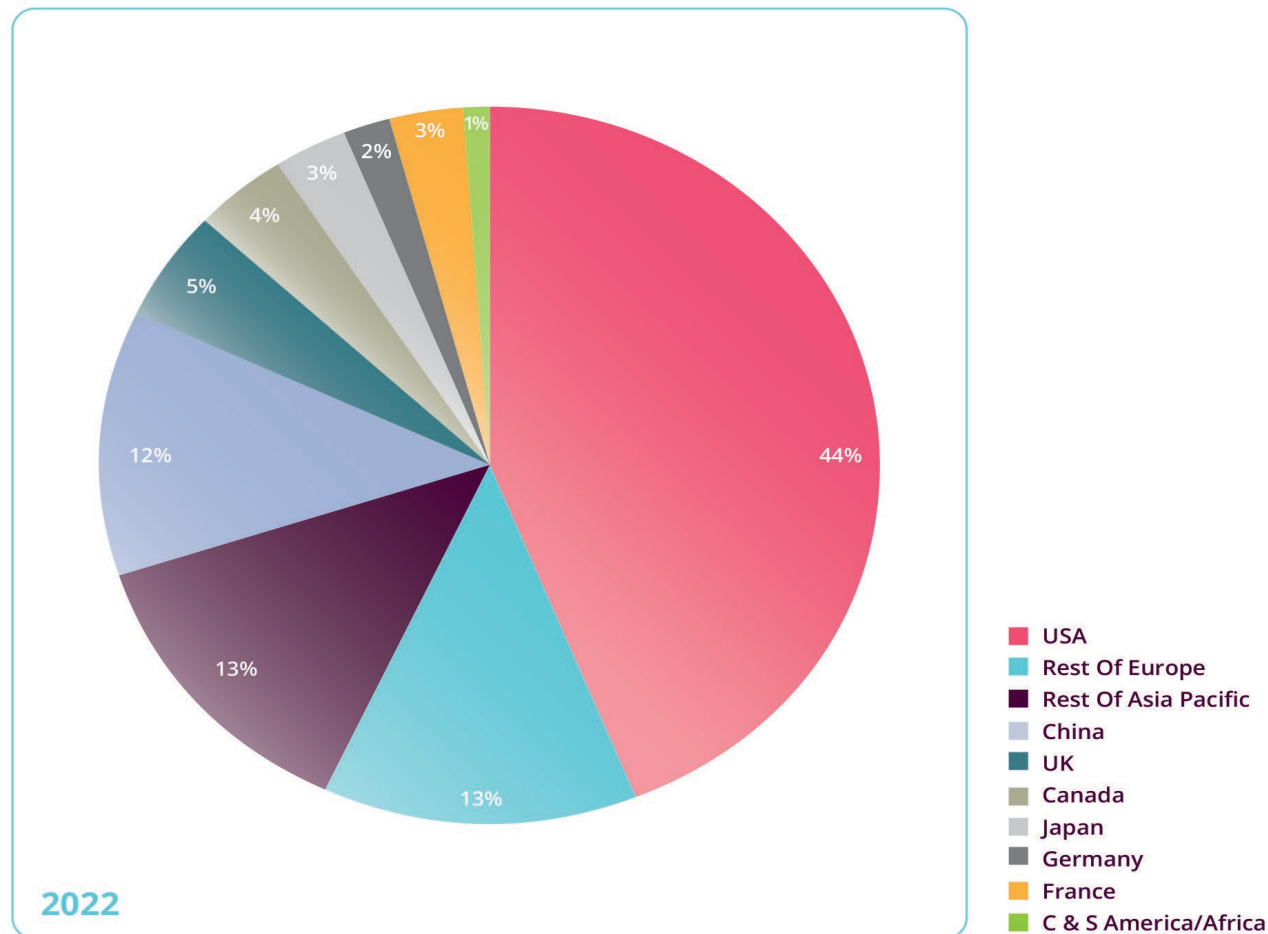
Share leaps up from 9% in 2021

- Number of companies up by 43.3% in a single year
- 792 in 2022 vs 522 in 2021

US and Europe cede ground

- US down 2% to 44%
- Rest of Europe down 1% to 13%

Distribution of R&D companies by HQ country/region, 2022



Over 20% of drugs in R&D in Chinese development

Second-most important market

A fifth of drugs are now in R&D in China

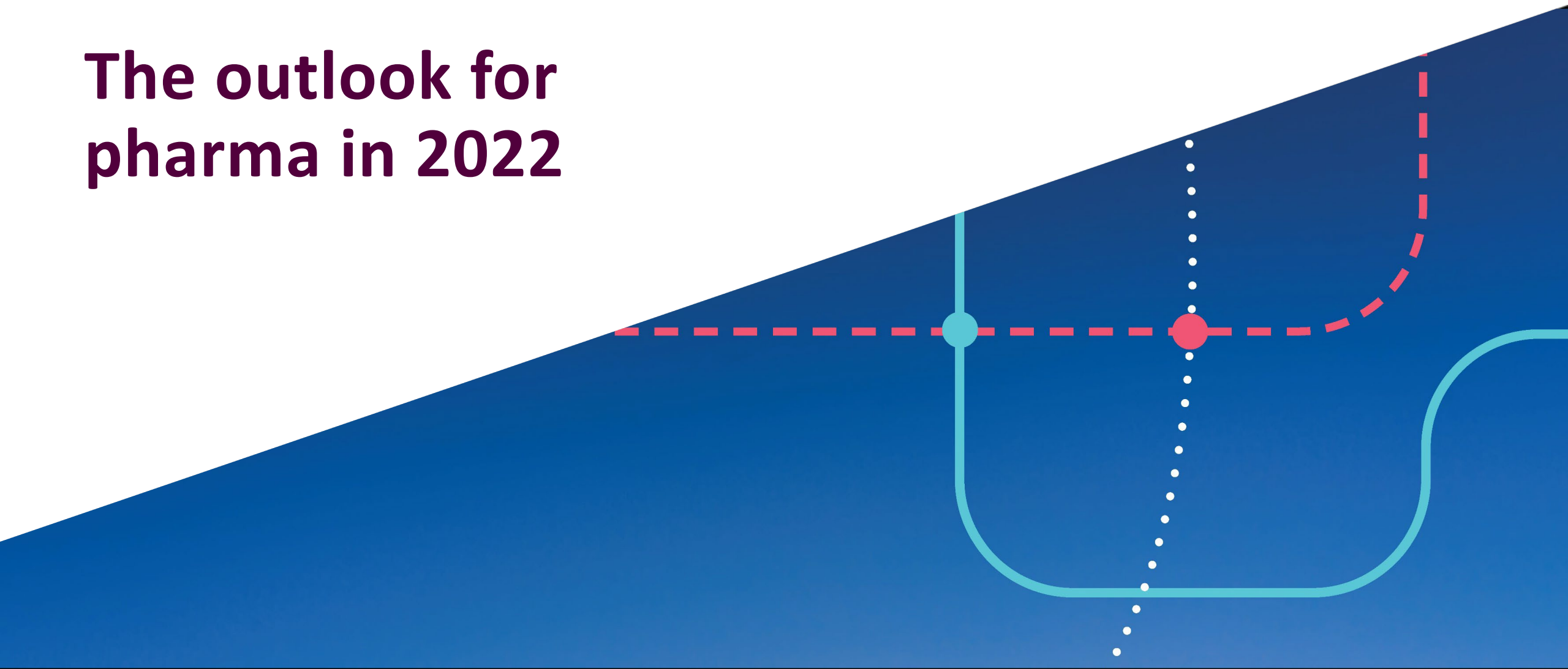
- US remains way out in front
- China moves into runner-up position ahead of UK



Where is R&D actually occurring?

COUNTRY	NO. OF DRUGS	% OF PIPELINE
US	10,736	53.4
China	4,189	20.8
UK	2,887	14.4
South Korea	2,627	13.1
Germany	2,299	11.4
Canada	2,182	10.9
France	2,057	10.2
Australia	2,010	10.0
Japan	1,931	9.6
Spain	1,909	9.5
Netherlands	1,680	8.4
Belgium	1,608	8.0
Italy	1,603	8.0
Poland	1,477	7.3
Sweden	1,402	7.0
Denmark	1,352	6.7
Switzerland	1,346	6.7
Hungary	1,258	6.3
Czech Republic	1,248	6.2
Austria	1,220	6.1
Taiwan, China	1,120	5.6
Ireland	1,086	5.4
Bulgaria	1,082	5.4
Finland	1,079	5.4
Portugal	1,032	5.1
Romania	1,026	5.1
Greece	1,018	5.1
Norway	1,014	5.0
Israel	1,006	5.0

The outlook for pharma in 2022



Notable approvals/launches in 2022 so far

Part 1



Idorsia Pharmaceuticals' Quviviq (daridoxerant)

- Approved in the US in January for insomnia
- A dual orexin receptor antagonist
- Competition for Merck & Co's rival dual orexin receptor antagonist Belsomra

IMMUNOCORE

Immunocore's Kimmtrak (tebentafusp)

- Approved in the US in January as the first drug for metastatic uveal melanoma
- First approved T-cell receptor (TCR) therapy
- Immunocore's first approved product



Roche's Vabysmo (farcimab)

- Approved in the US in January for wet AMD and diabetic macular edema
- Bispecific antibody targets and inhibits two disease pathways: Ang-2 and VEGF-A)
- Roche is playing the cost-effectiveness card with Vabysmo cf Eylea



Notable approvals/launches in 2022 so far

Part 2



Sanofi's Enjaymo (sutimlimab)

- Approved in the US in February for cold agglutinin disease
- Targets C1s and is meant to prevent destruction of red blood cells
- Sanofi acquired Enjaymo when it bought Bioverativ in 2018 for \$11.6bn



AgiOS Pharmaceuticals' Pyrukynd (mitapivat)

- Approved in the US in February for hemolytic anemia in adults with pyruvate kinase deficiency
- Pyruvate kinase stimulant
- Significant milestone for Agios, also in development for sickle cell and beta-thalassemia



Janssen and Legend Biotech's Carvykti (ciltacabtagene autoleucel)

- Approved in the US in February for multiple myeloma
- CAR-T therapy that targets B-cell maturation antigen (BCMA)
- Priced at a premium to BMS/2seventy Bio's Abecma – the first anti-BCMA CAR-T

Notable approvals/launches in 2022 so far

Part 3



CTI BioPharma's Vonjo (pacritinib)

- Accelerated approved in the US in February for myelofibrosis
- JAK2/IRAK1 inhibitor, CTI's first commercial product
- Unlike other JAK inhibitors, Vonjo's label does not carry a black box warning



Bristol Myers Squibb's Opdualag (relatlimab/nivolumab)

- Approved in the US in March for melanoma
- LAG-3 blocker relatlimab in a fixed-dose combination with Opdivo
- Third class of checkpoint inhibitor



Merck & Co/Kyorin's Lyfnua (gefapixant)

- Approved in Japan in January for refractory or unexplained chronic cough
- P2X3 receptor antagonist
- CRL from the US FDA in January



Notable approvals/launches so far in 2022

China



Jiangsu Hengrui Pharmaceuticals' AiRuiKang (dalpiciclib)

- Approved in China in January for HR+/HER2- advanced breast cancer
- First domestically developed CDK4/6 inhibitor to gain marketing approval in China
- Designed to reduce hepatotoxicity



Jiangsu Hengrui Pharmaceuticals' henagliflozin

- Approved in China in January for type 2 diabetes
- Sodium-glucose transport protein 2 (SGLT2) inhibitor
- Beijing's determination to prop up domestic pharma innovation



Henlix Biotech's Hansizhuang (serplulimab)

- Launched in March for advanced unresectable or metastatic MSI-H solid tumors
- Recombinant humanized anti-PD-1 monoclonal antibody
- Innovative product among a number of biosimilars for the firm



Notable approvals/launches expected in 2022

Part 1



Gilead Sciences' lenacapavir

- Longer-acting drug for HIV/AIDS - first-in-class HIV-1 capsid inhibitor
- Complete response letter received from the FDA - no new studies needed
- Could have use in PrEP and catalyze this market



Eli Lilly's tirzepatide

- Filed in US and EU for type 2 diabetes in October 2021
- First glucose-dependent insulinotropic polypeptide (GIP)/GLP-1 co-agonist
- Competing against entrenched once-weekly GLP-1 agonists



Sanofi's Xenpozyme (olipudase alfa)

- Recombinant sphingomyelinase enzyme for Niemann-Pick disease
- The only approved therapy globally for the rare genetic disorder
- Firm's first product to be approved under Japan's "sakigake" designation

Notable approvals/launches expected in 2022

Part 2



Roche's mosunetuzumab

- Filed in EU and US For follicular lymphoma
- CD20 x CD3 bispecific antibody
- Breakthrough therapy designation and EU accelerated assessment



Johnson & Johnson (Janssen)'s teclistamab

- Under review for relapsed or refractory multiple myeloma
- Bispecific antibody targeting B-cell maturation antigen (BCMA) and CD3
- FDA 20 July user fee goal – breakthrough therapy designation



Bristol Myers Squibb's mavacamten

- First-in-class cardiac myosin inhibitor for obstructive hypertrophic cardiomyopathy
- 28 April PDUFA date - breakthrough therapy designation
- Peak annual sales predicted at more than \$4bn

Concluding Thoughts



Pharma R&D seems to be going from strength to strength

- Pipeline growth accelerated in 2022
- More drugs and companies, more diseases and targets being studied
- Record for new drug launches in a single year smashed again



Key trends in 2021-2022

- COVID response continued, albeit at a lower level
- Rare disease R&D booming
- Cell and gene therapy continue to be hot
- China becoming a major pharma powerhouse



Positive outlook for 2022 as world returns to 'normal'

- 12 approvals for NASs to date from the FDA in Q1
- The impact of the COVID-19 era continues



Thank you for your attention

Questions?

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