

WHITE PAPER

# Annual Completed Clinical Trials Report

Pharma industry returns with vigorous growth and high success rates

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## Introduction

Trialtrove recorded 4,295 industry-sponsored clinical trials from Phase I through Phase III/IV that either reached completed status or reported primary endpoints during 2023.\* This number is 10.7% higher than the 3,881 completed trials in 2022 and reflects a return to growth by this metric. Another 1,030 trials were reported as terminated in 2023, representing an 8.1% increase over 2022 terminations (953). Since 2020, the completed trials counts have alternated between annual decreases, in 2020 and 2022, and increased trial completions, in 2021 and 2023.

In contrast with the pandemic period oscillations, all therapeutic areas (TAs) reported higher completions in 2023. Infectious diseases (ID) growth was negligible compared to other

TAs and central nervous system (CNS) returned to the top three TAs (third place) for the first time since 2020. The broad increase in annual trial completions reflects diminishing COVID-19 pandemic disruptions on clinical trial activity.

The high activity sponsored by the top 20 group of companies dominates topline trial counts again this year. All other pharma (AOP) companies continue to increase their share of completed trials activity while occupying certain geographic and disease focus niches. This white paper highlights how broad-based completed trials growth is accompanied by higher success rates across multiple TAs, as pandemic-related disruptions and infectious disease-related activity come to an end after three years of oscillations.



\*The snapshot of clinical trials completed between Jan. 1, 2023 and Dec. 31, 2023 was taken on Feb. 14, 2024. Industry sponsors are classified as "Industry, Top 20 pharma" or "Industry, all other pharma."

## Topline Trial Landscape Metrics

Oncology ranked number one for completed trial activity again in 2023 (Table 1). Autoimmune/inflammation (A/I) retained second rank, as it has for three of the past four years. CNS returned to third rank, following relatively small annual changes during the pandemic years (2020–22). During that period, ID activity surged and nudged CNS from the top three TAs. Metabolic/endocrinology (met/endo) returned to fourth place, narrowly edging

out ID. As ID drops in trial activity to fifth rank, it returns to its early pandemic period, 2020, position, albeit with far higher counts (648 vs. 485). Cardiovascular (CV) reversed its trend of decreasing trial completions (2020–22). Vaccines trial completions continue to grow and have nearly doubled since 2020. The four smallest TAs have retained the same rank order since at least 2016. Ophthalmology and genitourinary (GU) also grew modestly in 2023.

**Table 1.** Trial counts and rankings for completed trials, by year

Therapeutic area	Ranking				Trial Count <sup>a</sup>			
	2023	2022	2021	2020	2023	2022	2021	2020
Oncology	1	1	1	1	1,197	1,096	1,222	1,128
Autoimmune/Inflammation	2	2	3	2	758	669	700	665
CNS	3	4	4	3	678	619	620	617
Metabolic/Endocrinology	4	5	5	4	649	570	545	567
Infectious Diseases <sup>b</sup>	5	2	3	5	648	643	726	485
Cardiovascular	6	6	6	6	358	293	303	308
Vaccines	7	7	7	7	286	255	231	157
Ophthalmology	8	8	8	8	136	121	97	92
Genitourinary	9	9	9	9	62	58	62	66

<sup>a</sup> Trials may span multiple therapeutic areas

<sup>b</sup> Excludes Vaccines trials

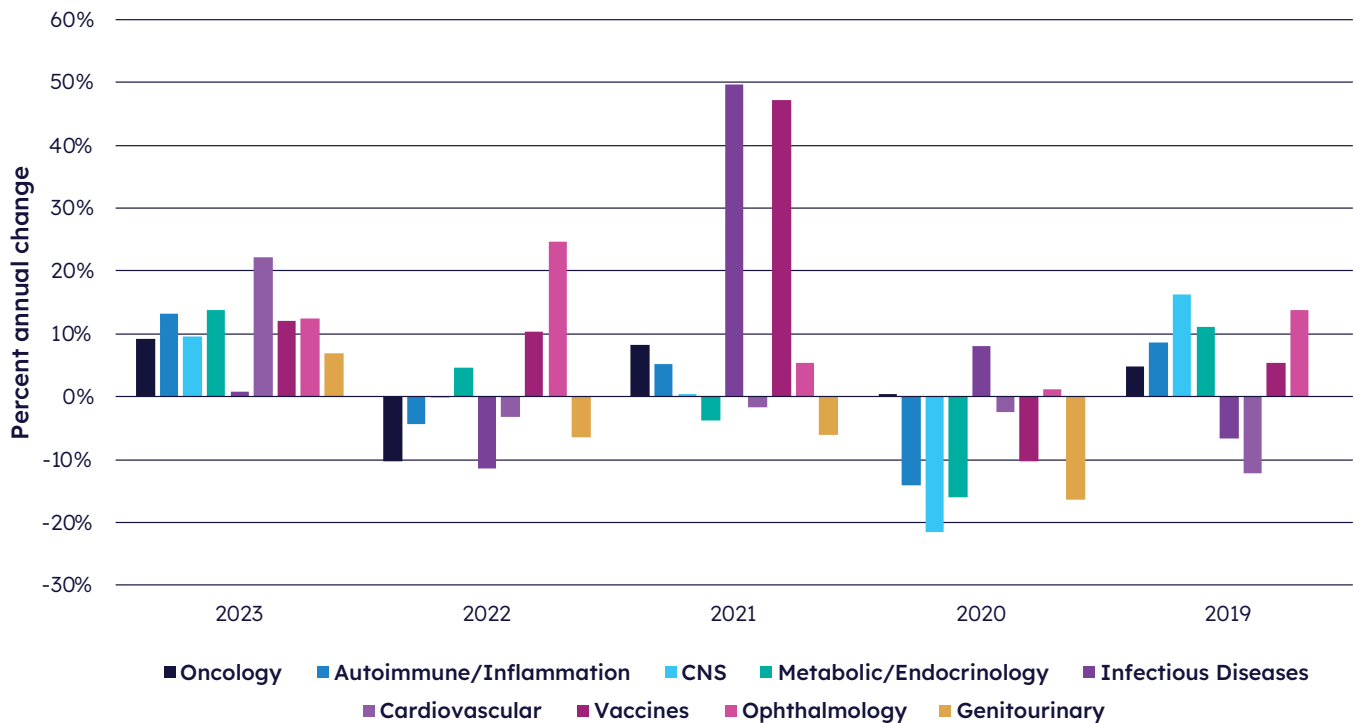
Source: Trialtrive, February 2024

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The cross-TA growth in 2023 trial activity is reminiscent of trends observed in the pre-pandemic years. The annual change rates for 2019 to 2023 (Figure 1) illustrate volatility across all TAs during the pandemic years, 2020-22, while recent broad-based growth was similar

to 2019 activity changes, when only ID and CV completions decreased relative to the prior year. It will be interesting to track whether recent surges in CV trial activity translate into meaningful developments in this TA.

**Figure 1.** Relative annual changes in completed trial counts, by therapeutic area



Source: Trialtrove, February 2024

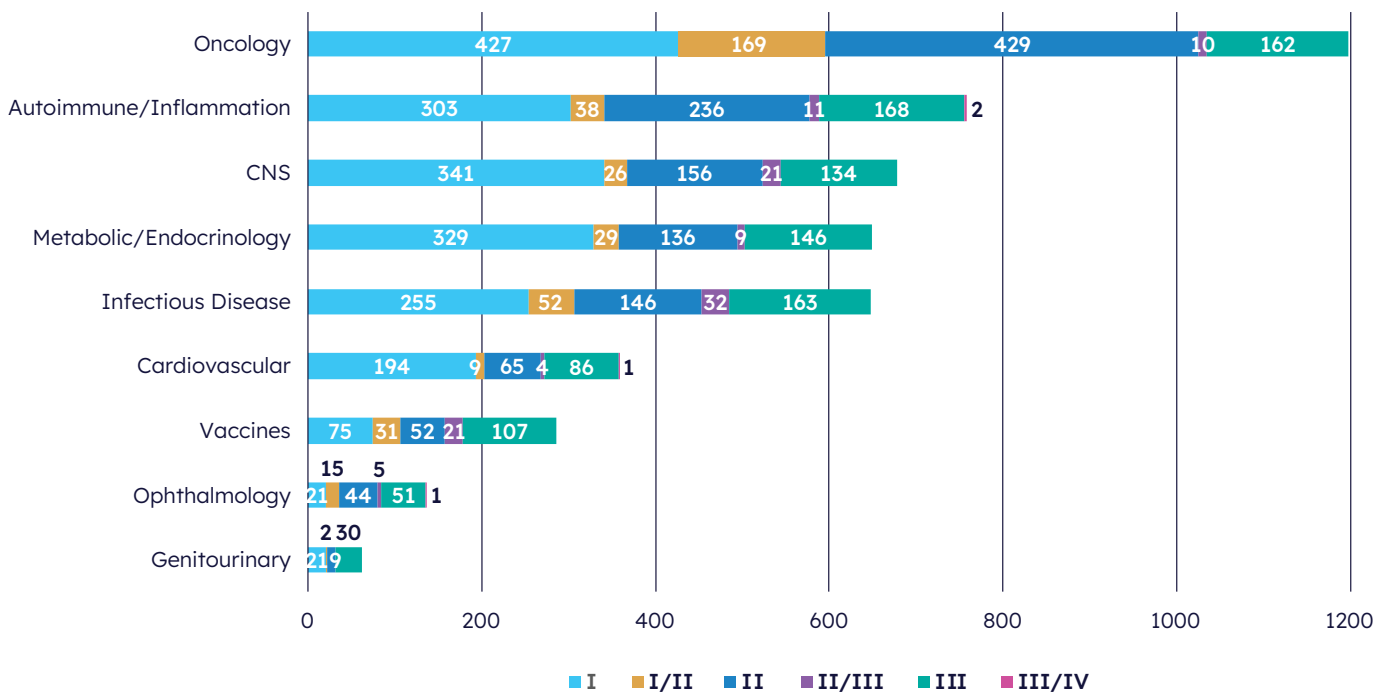
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## Distribution of 2023 completed trials by therapeutic area and phase

The distribution of trial counts by phase remains the same as that observed in our prior years' analyses, as later-phase counts (Phase II/III-III, III/IV) are typically lower for most TAs than the early phases, I-I/II (Figure 2). This is as expected, in part due to higher hurdles that must be met to advance into larger-scale, late-stage trials. Oncology continues to run a relatively high proportion of Phase I/II trials (14%). Vaccines and ophthalmology also completed above average (7%) Phase I/II trial activity, with 11% each. Efficacy endpoints are commonly evaluated in Phase I studies in

oncology (62%; 264 of 427), vaccines (95%; 71 of 77), and ophthalmology (33%; 7 of 31), compared with the mid-sized TAs, A/I (20%), CNS and met/endo (15% each), and CV (11%) (data not shown). These TA-specific observations reflect varied degrees of focus on early demonstration of efficacy. Annual growth driven by increased Phase I activity was most pronounced in CV (54% vs. 47%) and ID (39% vs. 33%), presumably from new candidates in clinical development. Phase I activity in A/I, CNS, and oncology changed, year-on-year, by only an average of 1.7%, which suggests growth in these TAs was mainly due to increased activity in later-stage trials as successful clinical stage drugs move forward.

**Figure 2.** Distribution of industry-sponsored trials completed in 2023, by therapy area and phase



Source: Trialtrove, February 2024



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The top five diseases for trial completions in 2023 (Table 2) were COVID-19, type 2 diabetes (T2D), non-small cell lung cancer (NSCLC), respiratory vaccines, and breast cancer. Trial activity for the top respiratory infection (COVID-19) has topped the disease list since 2021 and includes trials that cross over to respiratory vaccines, contributing to this broad indication's inclusion in the top five for the first time, at rank four. COVID-19 counts remain high compared to 2020, but a substantial

annual decrease is reported as this pandemic transitions to the endemic chapter. T2D ranked second while it moved closer its pre-pandemic first rank and contributed substantially to the annual growth observed for met/endo. Breast cancer and NSCLC consistently rank in the top five diseases. NSCLC's third rank was attained with counts that nearly match their high-water mark recorded in 2021. Breast cancer counts dropped to their lowest levels since 2016, and the disease is now ranked fifth.

**Table 2.** Top five diseases for trials completed in 2023, and comparison to prior three years

Disease	2023	2022	2021	2020
Novel coronavirus (2019-nCoV, COVID-19)	255 (1)	304 (1)	354 (1)	116 (9)
Type 2 diabetes	205 (2)	182 (2)	155 (5)	161 (4)
Non-small cell lung cancer	202 (3)	160 (5)	207 (2)	182 (1)
Respiratory vaccines	162 (4)	159 (6)	121 (-)	45 (-)
Breast cancer	155 (5)	174 (3)	164 (4)	165 (3)

Source: Trialtrove, February 2024

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## Trial success rates

Completed trials are assigned an outcome evaluation (positive, negative, unknown, or indeterminate) when those studies report safety, efficacy, or biomarker/surrogate efficacy

outcomes in the public domain. Success rates for diseases reporting highest numbers of successful trials were evaluated, and these diseases were ranked based as a proportion of their total completed trials in 2023 (Table 3).

**Table 3.** Diseases with  $\geq 25$  completed trials attaining primary endpoint

Disease	I	I/II	II	II/III	III	III/IV	Total	% of all Trials	Rank#
Novel coronavirus (2019-nCoV. COVID-19)	23	11	15	10	20	0	79	31.0%	11
Type 2 Diabetes	21	2	10	3	29	0	65	31.7%	8
Non-small cell lung cancer	9	4	31	1	18	0	63	31.2%	9
Respiratory Vaccines	17	9	8	6	21	0	61	37.7%	3
Breast cancer	16	7	14	1	9	0	47	30.3%	12
Lymphoma. Non-Hodgkin's*	6	5	26	0	1	0	38	31.1%	10
Dyslipidemia	6	0	9	0	17	0	32	33.3%	6
Atopic Dermatitis	6	2	10	0	13	0	31	34.1%	4
Alzheimer's Disease	17	1	5	1	7	0	31	39.7%	1
Obesity	10	1	9	1	8	0	29	33.0%	7
Depression	16	3	6	0	3	0	28	38.9%	2
Colorectal cancer	10	4	11	0	2	0	27	26.7%	13
Liver cancer*	5	7	11	0	2	0	25	33.8%	5

\*Rare disease

# Rank based on percentage of trials attaining primary outcome per disease

Source: Trialtrive, February 2024

The number of diseases (13) that completed at least 25 successful trials was the same for two years in a row. The top five diseases by overall completed trial counts (Table 2) were among those reporting at least 25 positive trials. COVID-19 (respiratory infections) returned the highest number of positive trials again this year, but those counts continue to decrease, year over year, 79 vs. 94. Its rank also dropped from eighth to 11th. Reported T2D positive trials (65)

nearly doubled compared with 2022 (34) and ranked eighth. NSCLC reported more positive trials, 63 vs. 54, and ranked ninth. Respiratory vaccines reported more successful trials, 61 vs. 53, but moved from first to third rank. The vast majority of these trials (40 of 61) were COVID-19 vaccines and included 10 pivotal studies, both for first approvals and expanded indications. Breast cancer reported fewer successful trials, 47 vs. 54, and dropped from rank seven to

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12. Non-Hodgkin's lymphoma (NHL) reported more positive trials, 38 vs. 30, while retaining the 10th rank. Six diseases that achieved  $\geq 25$  positive trials last year did not attain those numbers in 2023, including respiratory infections (non-COVID-19), esophageal cancer, gastric cancer, melanoma, diabetic complications, and psoriasis. In their places are dyslipidemia (32 trials; rank six), atopic dermatitis (31 trials; rank four), Alzheimer's disease (AD; 31 trials; rank one), obesity (29 trials; rank seven), depression (28 trials; rank two), and liver cancer (25 trials; rank five).

The average success rate for these top diseases increased from 31.6% to 33.3% year over year. Historically, CNS diseases have not ranked among the more successful indications. The

top rankings for AD and depression are driven in large part by high numbers of positive Phase I trials. As noted previously, only about 15% of CNS and met/endo Phase I trials evaluate efficacy endpoints. Therefore, positive Phase I trials in the CNS and met/endo diseases, featured in Table 3, were further reviewed to assess the basis for these positive determinations. For AD and depression, positive Phase I outcomes were assigned mainly based on achieving early safety evaluations. However, a few subjective efficacy evaluations and surrogate efficacy/biomarker measures, including biomarkers of target modulation and EEG measurements, also were reported. Most of the positive Phase I obesity and T2D trials evaluated either classical or surrogate efficacy endpoints, rather than safety alone.





## Leading Trial Sponsors

Shifting our focus to top sponsors, a similar analysis identifies the most active and successful sponsors of 2023. Our completed trials analysis focuses on trials of both top 20 pharma, based on sales data in the annual *In Vivo Outlook 2024* dataset,<sup>1</sup> and a separate group comprising AOP, excluding generics-only companies. The top five sponsors by overall completed trial counts in 2023 are again entirely comprised of top 20 pharma (Table 4).

Pfizer achieved the top rank again this year. AstraZeneca (AZ) has held second rank for the past three years. Merck & Co. and Roche reported their highest completed trials counts in five years, attaining third and fourth ranks, respectively. Bristol Myers Squibb (BMS) rounds out the top five by placing at rank five. Novartis dropped from the top five this year and has completed fewer trials annually for most of the past five years.

**Table 4.** Top five sponsors\* completing trials in 2019–23

Sponsor	2023 (rank)	2022 (rank)	2021 (rank)	2020 (rank)	2019 (rank)
Pfizer	174 (1)	165 (1)	134 (4)	152 (2)	137 (6)
AstraZeneca	170 (2)	159 (2)	175 (2)	142 (3)	179 (1)
Merck & Co.	152 (3)	116 (4)	140 (3)	127 (6)	125 (7)
Roche	150 (4)	116 (4)	115 (7)	138 (5)	149 (4)
Bristol-Myers Squibb	148 (5)	142 (3)	177 (1)	139 (4)	141 (5)

\*Trial count includes co-sponsored trials

Source: Trialstrove, February 2024

Beyond the top five, the following sponsors completed 50 or more trials: Johnson & Johnson (J&J; 133), Roche (120), Eli Lilly (110), Novartis (92), Sanofi and GSK (77 each), Boehringer Ingelheim (BI; 74), AbbVie (73), Jiangsu Hengrui Pharma (72), Amgen (56), Novo Nordisk (52), and Takeda (51). These sponsors also are top 20 pharma who exceeded their 2022 trial

completions. Other top 20 pharma completed fewer than 50 trials during this period, including Gilead, Bayer, Moderna, BioNTech, and Viatrix. Top 20 pharma activity increased over the prior year, 1,561 vs. 1,420. However, the trend towards lower participation rates compared with AOP (36% vs. 67%)<sup>2</sup>, was unchanged in 2023 (data not shown).

1. In Vivo; Outlook; Scrip 100. Available from: [https://invo.citeline.com/-/media/editorial/in-vivo/2023/12/iv2312\\_scrip-100-infographic-2024.jpg?rev=12d02f368af440a2b14c810f5aada908&hash=99761F7864BA229EBB4B594A54E60461](https://invo.citeline.com/-/media/editorial/in-vivo/2023/12/iv2312_scrip-100-infographic-2024.jpg?rev=12d02f368af440a2b14c810f5aada908&hash=99761F7864BA229EBB4B594A54E60461) [Accessed March 19, 2024].

2. 2022 Completed Trials: A Roller Coaster Ride for Industry Sponsors. Available from <https://www.citeline.com/en/resources/2022-completed-trials-a-roller-coaster-ride-for-sponsors> [Accessed March 19, 2024].

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The performance of industry sponsors was assessed by their overall counts of successful trials (Table 5). The number of sponsors reporting at least 25 positive trials increased, year over year, from seven to 11. Six of last year's most successful sponsors remained on this list. Novartis did not meet this criterion in

2023, while Eli Lilly, Sanofi, and AbbVie met the mark this year, too. The highest performer (AstraZeneca) reported near or greater than twofold higher positive trial counts (64) than the last four sponsors by this measure, including Eli Lilly (33), Sanofi (29), Novartis, and AbbVie (28 each).

**Table 5.** Companies attaining primary endpoints in >25 trials, by phase

Sponsor	I	I/II	II	II/III	III	III/IV	Total
AstraZeneca	12	5	21	1	17	0	64
Merck & Co.	9	3	18	1	14	0	47
Bristol-Myers Squibb	7	6	19	1	10	0	43
Pfizer	13	3	15	0	12	0	43
Johnson & Johnson	5	1	18	1	12	0	37
Jiangsu Hengrui Pharma	12	1	13	2	7	0	35
Roche	5	0	16	0	13	0	34
Eli Lilly	11	1	5	0	16	0	33
Sanofi	7	0	8	0	14	0	29
Novartis	3	1	10	0	14	0	28
AbbVie	5	1	9	1	11	1	28

Source: Trialtrove, February 2024



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Sponsors completing at least 40 trials were assessed for success rates, calculated as a percentage of their total trials (Table 6). The number of sponsors meeting this mark (15) dropped from 17 in the past two years. The highest success rate was achieved by Jiangsu Hengrui Pharma (51.4%), whose trials are mainly run exclusively in China. The lowest rate was observed for BI (13.5%). This year's top rate is substantially higher than those achieved in the prior two years, 32.4% and 42.9%. The average rate across these sponsors also increased, 31.3% vs. 24.7%. Six of these sponsors performed

above the average rate, while nine fell below it.

Success rates reflect several factors, including whether sponsors actually report interpretable results during this period and the impact of Phase I trials that often have no efficacy/surrogate efficacy outcomes to count as positive or negative. Most top sponsors returned higher success rates in Phase III than they achieved as an average of their totals (Table 6). This reflects more efficacy outcomes reported for that phase and eliminates the Phase I factor from the calculation.

**Table 6.** Percentage of trials\* attaining primary endpoint, by phase, for companies completing >40 trials in 2023

Sponsor	I	I/II	II	II/III	III	III/IV	Total
Jiangsu Hengrui Pharma	30.0%	0.0%	73.3%	100.0%	59.1%	0.0%	51.4%
AbbVie	20.8%	12.5%	75.0%	100.0%	40.7%	100.0%	38.4%
Sanofi	41.2%	0.0%	38.1%	0.0%	45.2%	0.0%	37.7%
AstraZeneca	21.1%	60.0%	44.1%	66.7%	63.6%	0.0%	37.6%
Amgen	20.0%	50.0%	38.9%	0.0%	40.0%	0.0%	33.3%
Merck & Co.	20.9%	20.0%	42.0%	50.0%	33.3%	0.0%	31.6%
Novartis	21.4%	14.3%	29.4%	0.0%	38.9%	0.0%	30.4%
Eli Lilly	21.2%	100.0%	16.7%	0.0%	59.3%	0.0%	30.0%
Bristol-Myers Squibb	15.9%	27.3%	32.8%	100.0%	43.5%	0.0%	29.1%
GlaxoSmithKline	14.3%	25.0%	40.0%	0.0%	37.9%	0.0%	28.6%
Roche	20.0%	0.0%	34.8%	0.0%	37.1%	0.0%	28.3%
Johnson & Johnson	17.2%	11.1%	34.6%	25.0%	30.8%	0.0%	27.8%
Takeda	21.4%	50.0%	17.6%	0.0%	25.0%	100.0%	27.5%
Pfizer	17.3%	25.0%	31.3%	0.0%	36.4%	0.0%	24.7%
Boehringer Ingelheim	2.5%	0.0%	37.5%	100.0%	15.4%	0.0%	13.5%

\*Trial count includes co-sponsored trials

Source: Trialtrive, February 2024

## Top Three Therapeutic Areas: Assessment by Top Sponsors, Diseases with Positive Pivotal Trials, and Pipeline Therapeutics

The top three TAs for completed trial counts in 2023 are oncology, A/I, and CNS. Oncology and A/I have completed sufficient trials to make this list for all eight years of our analyses. The COVID-19 pandemic has driven high trial activity for ID during the past two years which nudged CNS to a lower ranking. A granular analysis for these highest activity TAs provides insights into their top sponsors, top diseases, and the positive novel candidates reported in 2023.

### Oncology: Top sponsors, indications, and pipeline drugs in successful pivotal trials

The top 10 sponsors within oncology are ranked by completed trial counts, assessed for success rates across all trial phases and for their counts of positive pivotal trials (Table 7). Most of the same sponsors returned to this list in 2023. The exceptions are Innovent, the only AOP sponsor on this list, and a company that runs mainly Chinese-only studies, which joined at ninth rank, while AbbVie did not return this level of success in oncology in 2023. BMS attained first rank for the past three years. BMS's count of oncology trials, 108, is fourfold higher than the two lowest ranked sponsors' counts, Innovent (27) and Amgen (26). Merck & Co. moved from fourth to second rank, while both AZ and Roche moved down, to third and fourth rank, respectively. Pfizer and J&J reported higher positive

oncology counts. Novartis dropped from 46 to 30 trials and from sixth to eighth rank. J&J moved up to sixth from ninth rank, with higher positive counts, 36 vs. 25, as Jiangsu Hengrui Pharma and Amgen rose to rank seventh and 10th, respectively.

Oncology top sponsors collectively improved their success rates in 2023: Merck & Co. (33.3% vs. 30.0%), AstraZeneca (51.1% vs. 25.6%), Pfizer (39.3% vs. 26.4%), J&J (50.0% vs. 16.0%), Jiangsu Hengrui Pharma (54.3% vs. 53.1%), Novartis (36.7% vs. 28.3%), and Amgen (38.5% vs. 33.3%). BMS returned comparable success rate, year over year, 33.3% vs. 34.6%, and reported the highest number of positive pivotal trials (seven). Merck & Co. (six), J&J and AZ (five each), Pfizer (four), and Roche (three) also reported notable counts of positive pivotal trials. Novartis and Amgen each reported positive results for two pivotal trials during this period. Both Chinese sponsors, Jiangsu Hengrui Pharma and Innovent, reported a single pivotal trial and these were run only in China.

The sum of top sponsors' trial counts (567) is 9% higher than the 2022 count (518). In addition, the average success rate, 38.9%, across oncology's top sponsors improved over the 2022 average, 28.8%. Taken together, the return to growth in 2023 is accompanied by increased success rates for top sponsors in oncology.



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**Table 7.** Top 10 sponsors with completed oncology trials, by success rates and positive pivotal trial counts

Sponsor	Negative outcome/ primary endpoints not met	Outcome indeterminate	Outcome unknown	Early positive outcome	Positive outcome/ primary endpoints met	NA	Total Trials (rank)	Success Rate	Positive Pivotal Trials (count)
Bristol-Myers Squibb	13	13	43	1	35	3	108 (1)	33.3%	7
Merck & Co.	12	12	34	0	30	2	90 (2)	33.3%	6
AstraZeneca	9	4	29	1	44	1	88 (3)	51.1%	5
Roche	10	11	30	0	19	1	71 (4)	26.8%	3
Pfizer	4	10	17	0	22	3	56 (5)	39.3%	4
Johnson & Johnson	2	5	11	0	18	0	36 (6)	50.0%	5
Jiangsu Hengrui Pharma	1	0	14	0	19	1	35 (7)	54.3%	1
Novartis	2	1	14	1	10	2	30 (8)	36.7%	2
Innovent Biologics	0	0	19	1	6	1	27 (9)	25.9%	1
Amgen	1	3	12	0	10	0	26 (10)	38.5%	2

Note: Indeterminate designation is assigned to trials when the outcome is neither clearly positive nor negative. Unknown is assigned to trials that have yet to report full results for their primary endpoints. NA indicates trials with no efficacy/safety outcomes to evaluate.

Source: Trialtrive, February 2024

At the disease level, successful pivotal studies spanned 25 oncology diseases (Figure 3). These trials are tagged either as registration for first approvals (46) or expanded indication for previously approved drugs (37). This tally of successful pivotal trials (83) is higher than the 2022 number (72) but closely matches the 2021 count (84). Many of these trials include patients

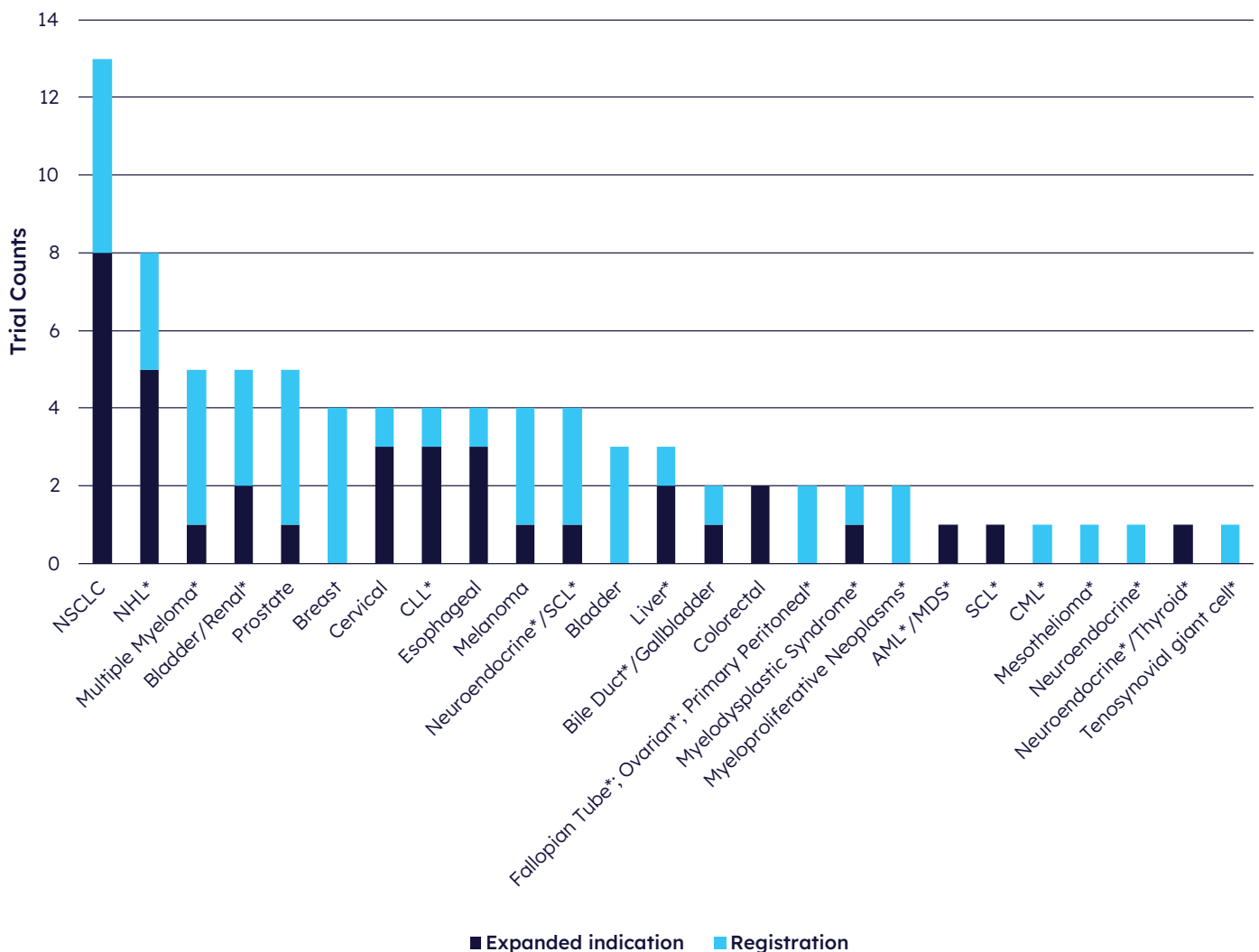
with a rare disease (20 rare diseases in total). NSCLC remains the top disease for the fourth year in a row with 13 positive pivotal NSCLC trials, compared with 11 in 2022. These NSCLC trials are predominantly expanded indication studies (eight out of 13). NHL counts (eight) have been the same for the past three years. Other diseases with stable counts year over year

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include NHL, prostate cancer, breast cancer, and melanoma. Counts for neuroendocrine and SCL pivotal trials increased in 2023. Trial activity for hematological cancers (myelodysplastic and proliferative neoplasms, leukemias, and lymphomas) collectively reported 11 positive pivotal trials, which is approximately twofold

lower than the 25 positive trials reported in 2022. Top 20 pharma sponsors, excluding Jiangsu Hengrui Pharma, sponsored 33 of these successful pivotal trials, and these trials were run globally with an average of 22 participating countries (data not shown).

**Figure 3.** Pivotal oncology trials achieving primary endpoints, by disease and filing type



\*Rare disease

ALL = acute lymphoblastic leukemia; AML = acute myelogenous leukemia; CLL = chronic lymphocytic leukemia; CML = chronic myelogenous leukemia; MDS = myelodysplastic syndrome; NHL = non-Hodgkin's lymphoma; NSCLC = non-small cell lung cancer; SCL = small cell lung

Source: Trialtrave, February 2024

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A further analysis identified the pipeline status drugs from positive pivotal oncology studies and assigned them as novel or not, based on whether theirs is a previously proven MOA (Table 8). There are six drugs with novel MOAs, as determined from their Pharmaprojects profiles. Two novel Roche candidates were successful in esophageal cancer, including a TIGIT receptor antagonist and an oncolytic adenovirus, whose Phase II pivotal Japanese trial reported success. The pipeline drugs with proven MOAs (22) are considered “me-too” candidates when prior approvals exist for the same MOA in

that disease. The proven MOAs in 2023 include familiar multiple protein kinase inhibitors, PARP inhibitors, DNA replication inhibitors, apoptosis stimulants, and others. Many Chinese sponsors reported positive outcomes for “me-too” drug trials that ran solely in China and will likely be filed only there rather than globally. The top 20 pharma sponsors that dominate oncology trial activity (Table 6) and reported positive pivotal trials are underrepresented in the analysis of pipeline status drugs (Table 8), indicating that most of their 2023 pivotal trials were for previously approved drugs.

**Table 8.** Pipeline status drugs in successful pivotal oncology trials, by sponsor, disease, MOA, and MOA novelty

Drug name	Sponsor	Disease	Mechanism of action	Novel
pelabresib	MorphoSys/ Constellation Pharma	Myeloproliferative Neoplasms*	BET protein inhibitor	Yes
Nidlegy	Philogen	Melanoma	IL-2 antagonist/TNFalpha agonist	Yes
BVAC-C	Cellid	Cervical	IL-7 agonist; T cell stimulant	Yes
pegargiminase	Polaris Group	Mesothelioma*	pegylated argiminase	Yes
tiragolumab	Roche	Esophageal	TIGIT R antagonist	Yes
suratadenoturev	Roche	Esophageal	oncolytic adenovirus	Yes
abiraterone acetate, Tavanta Therapeutics	Tavanta Thera	Prostate	17,20 lyase inhibitor	No
mitomycin-C (high dose), UroGen	UroGen Pharma	Bladder	DNA inhibitor	No
vimseltinib	Deciphera Pharma	Tenosynovial giant cell*	CSF1-R PKI	No
tarlatamab	Amgen	Neuroendocrine*; SCL*	CD3 agonist	No
lerociclib	Genor Biopharma	Breast	CDK 4/6 inhibitor	No
trastuzumab vedotin	Lepu Biopharma	Bladder; Renal*	Tubulin polymerization inhibitor	No

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tunlmetinib	KeChow Pharma	Melanoma	MEK1/2 inhibitor	No
patritumab deruxtecan	Daiichi Sankyo	Breast	DNA topoisomerase I inhibitor	No
benmelstobart	Sino Biopharma	Neuroendocrine*; SCL*	PD-L1 antagonist	No
zanidatamab	BeiGene	Bile Duct*; Gallbladder	ErbB-2 antagonist	No
senaparib	Impact Thera	Ovarian*	PARP-1 inhibitor	No
felzartamab	I-Mab Biopharma	Multiple myeloma*	CD38 antagonist	No
deutenzalutamide	Haisco Pharma	Prostate	Androgen R antagonist	No
retifanlimab	Incyte Corp	Neuroendocrine*; SCL*	PD-1 antagonist	No
abiraterone + niraparib	Johnson & Johnson	Prostate	17,20 lyase inhibitor; PARP1/2 inhibitor	No
trastuzumab, Chia Tai Tianqing Pharma	Chia Tai Tianqing Pharma	Breast	ErbB-2 antagonist	No
adebrelimab	Jiangsu Hengrui Pharma	Neuroendocrine*; SCL*	PD-1 antagonist	No
goserelin acetate, Luye Pharma	Luye Pharma	Prostate	LHRH agonist	No
zorifertinib	Alpha Biopharma	NSCLC	EGF R PKI	No
imetelstat	Geron	Myeloproliferative Neoplasms*; MDS*	Telomerase inhibitor	No
navitoclax	AbbVie	Myeloproliferative Neoplasms*; MDS*	Bcl-XL/2 inhibitor	No
resminostat	Yakult Honsha/4SC	NHL*	Histone deacetylase inhibitor	No

\*Rare disease

Gray fill: China-only trial

NHL = non-Hodgkin's lymphoma; NSCLC = non-small cell lung cancer; PKI = protein kinase inhibitor; R = receptor; SCL = small cell lung

Source: Trialstrove and Pharmaprojects, February 2024



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## Autoimmune/inflammation: Top sponsors, indications, and pipeline drugs in successful pivotal trials

The top 10 sponsors for A/I were likewise ranked by completed trial counts and assessed for success rates across all trial phases, and counts of their positive pivotal trials were tallied (Table 9). Most top sponsors in A/I are top 20 pharma, except for Incyte and Vertex. The rank order for returning sponsors changed. Many moved up in rank as sponsors reported more positive trials, including first- and second-ranked Eli Lilly (28 vs. 18) and BI (27 vs. 22). BMS and AbbVie, who are tied for third rank (26

trials each) compared with their tied fourth rank in 2022, also reported more positive trials in 2023. Sanofi moved to fourth from eighth rank, and J&J moved to fifth from sixth rank, both with year-over-year increases in positive trials. Sponsors that dropped this year include Pfizer, fifth vs. first rank, which reported fewer positive trials (23 vs. 29). Likewise, Novartis dropped rank to six from two with 21 vs. 26 trials. Amgen and GSK retained their rank at ninth and 10th, respectively, while reporting lower positive trials in 2023, Amgen (14 vs. 16) and GSK (10 vs. 14). AZ completed 20 positive A/I trials in 2022 and 2023, and dropped to rank seven vs. fifth, as other sponsors performed better.

**Table 9.** Top 10 sponsors with completed autoimmune/inflammation trials, by success rates and positive pivotal trial counts

Sponsor	Negative outcome/primary endpoints not met	Outcome indeterminate	Outcome unknown	Early positive outcome	Positive outcome/primary endpoints met	NA	Total Trials (rank)	Success Rate	Positive Pivotal Trials (count)
Eli Lilly	1	7	13	0	5	2	28 (1)	17.9%	2
Boehringer Ingelheim	0	4	10	0	4	9	27 (2)	14.8%	1
AbbVie	0	4	9	0	9	4	26 (3)	34.6%	3
Bristol-Myers Squibb	1	1	11	0	6	7	26 (3)	23.1%	0
Sanofi	1	0	12	1	9	1	24 (4)	41.7%	4
Johnson & Johnson	1	1	18	0	3	0	23 (5)	13.0%	0
Pfizer	0	2	13	0	6	2	23 (5)	26.1%	0
Novartis	2	3	10	0	4	2	21 (6)	19.0%	2
AstraZeneca	0	3	11	0	4	2	20 (7)	20.0%	0
Incyte	0	1	7	0	7	1	16 (8)	43.8%	2
Vertex	0	3	7	0	5	1	16 (8)	31.3%	2
Amgen	1	2	5	0	6	0	14 (9)	42.9%	1
GlaxoSmithKline	0	1	3	0	5	1	10 (10)	50.0%	1

Note: Indeterminate designation is assigned to trials when the outcome is neither clearly positive nor negative. Unknown is assigned to trials that have yet to report full results for their primary endpoints. NA indicates trials with no efficacy/safety outcomes to evaluate.

Source: Trialtrove, February 2024

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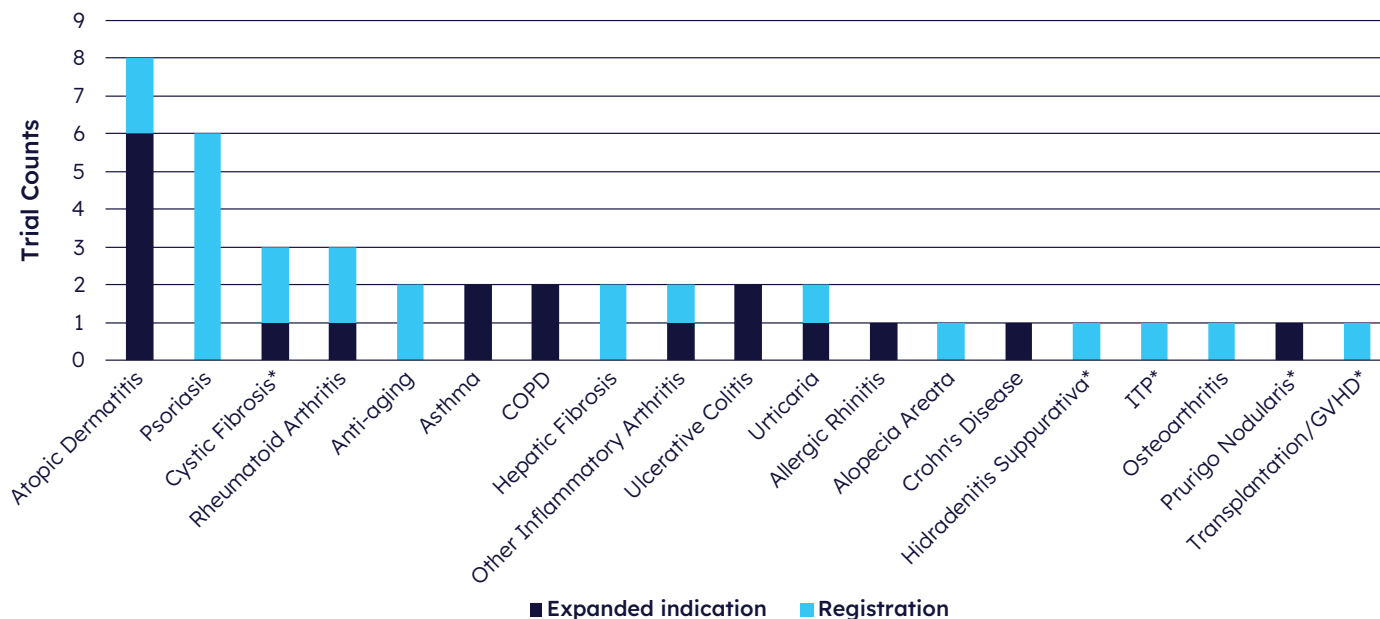
GSK was the lowest ranked top sponsor by overall positive trial counts but was number one by success rating, having achieved 50%.

The sum of top A/I sponsors' trial counts (274) is 23% higher than in 2022 (223). The average success rate for these companies, 28%, is higher than the top A/I sponsors attained in 2022 (20.8%). As noted for oncology top sponsors, the completed trials growth in 2023 is also reflected in higher counts of positive trials and higher success rates. Top sponsors reported positive outcomes for a total of 18 pivotal trials. By this measure, Sanofi, with four such trials, has the highest chance of further drug approvals because these are all expanded indication trials.

During this period, positive pivotal trials were reported for 19 diseases in the A/I portfolio (Figure 4). The tally of individual pivotal trials (42) includes 23 registration and 19 expanded indication trials, and exactly matches the tallies of both 2022 and 2021. Atopic dermatitis

had the highest counts with six expanded indication and two registration trials and surpassed last year's top disease, psoriasis, where performance was still high with six registration trials reported in 2023. Beyond these high-activity dermatology diseases, nine diseases reported at least two such trials. Large indications with robust clinical trials activity over many years, including RA, asthma, COPD, other inflammatory arthritis (psoriatic arthritis and spondyloarthropathies), ulcerative colitis, Crohn's disease, and allergic rhinitis, reported mainly on expanded indication trials. Positive trials in five rare diseases were mainly registration trials. Top A/I company Vertex, which specializes in cystic fibrosis therapies, sponsored all three of these pivotal trials. It should be noted that graft versus host disease (GvHD) is a rare condition that is indexed within transplantation, currently. The positive transplantation/GvHD registration trial was for chronic GvHD treatment.

**Figure 4.** Pivotal autoimmune/inflammation trials achieving primary endpoints, by disease and filing type



\*Rare disease

Source: Trialtrave, February 2024

# Annual Completed Clinical Trials Report

The pipeline status A/I drugs that achieved primary endpoints in pivotal trials are captured in Table 10, alongside evaluations of their MOA novelty. Three of 15 drugs have a novel MOA, while another 11 are “me-too” therapies and there is a single non-specified MOA, in alopecia areata, Coacillium.

Dermatology diseases continue to dominate A/I development, with nine such pivotal studies. The MOAs associated with these drugs include protein kinase inhibitors, cytokine antagonists, and an acetylcholine release inhibitor. Several novel cytokine signaling antagonists, including axatilimab, imsidolimab, and suciraslimab, are AOP-sponsored programs.

**Table 10.** Pipeline status drugs in successful pivotal autoimmune/inflammation trials, by sponsor, disease, MOA, and MOA novelty

Drug name	Sponsor	Disease	Mechanism of action	Novel
axatilimab	Syndax/Incyte	Transplantation/GvHD*	CSF-1 R antagonist	Yes
imsidolimab	AnaptysBio	Psoriasis	IL-36 R antagonist	Yes
suciraslimab	SinoMab BioScience	Rheumatoid Arthritis	CD22 antagonist	Yes
sonelokimab	Moon Lake Immuno thera	Hidradenitis Suppurativa*	IL-17A/IL-17F antagonist	No
SSGJ-608	3SBio	Psoriasis	IL-17A antagonist	No
botulinum neurotoxin serotype E	AbbVie/Allergan	Anti-Aging (dermatology)	Acetylcholine release inhibitor	No
ebdarokimab	Akeso Biopharma	Psoriasis	IL-12/IL-23 antagonist	No
remibrutinib	Novartis	Urticaria	Bruton PKI	No
sovleplenib	Hutchmed	ITP*	Syk TKI	No
rademikibart	Connect Biopharm	Atopic Dermatitis	IL-4 R antagonist	No
ustekinumab, Dong-A	Dong-A	Psoriasis	IL-12 antagonist	No
ivarmacitinib	Jiangsu Hengrui Pharma	Ankylosing spondylitis	JAK1 inhibitor	No
dexamethasone, Taiwan Liposome Company	Taiwan Liposome Company	Osteoarthritis	Glucocorticoid agonist	No
fazirsiran	Takeda/Arrowhead Pharma	Hepatic Fibrosis	A1AT inhibitor	No
Coacillium (Topical)	Legacy Healthcare	Alopecia Areata	Unknown/Natural product	N/A

\*Rare disease

Gray fill: China-only trial

A1AT = alpha-1 antitrypsin; CSF-1 = colony stimulating factor 1; GvHD = graft-versus-host disease; ITP = immune thrombocytopenia; R = receptor; TKI = tyrosine kinase inhibitor

Source: Trialrove and Pharmaprojects, February 2024

# Annual Completed Clinical Trials Report

## CNS diseases: Top sponsors, indications, and pipeline drugs in successful pivotal trials

The top CNS sponsors include seven top 20 pharma and five AOPs, as multiple sponsors returned the same number of completed trials (Table 11). CNS has typically included more AOP sponsors than top 20 pharma. From top-ranked AstraZeneca (20 positive trials) to last-ranked Biogen (nine), there is a twofold difference in positive trial counts. The success rates illustrate more broadly how programs are faring. By this metric, Otsuka (37.5%), AbbVie (45.5%), Roche

(33.3%), and Vertex (33.3%) returned the highest ratings.

The sum of these top CNS sponsor trials (170), and the average success rate, 20.8%, is lower than the other top TA metrics, as we typically observe for the broad and challenging CNS disease portfolio. The seven positive pivotal trials are a combination of new registrations and expanded indications. Many top CNS sponsors did not report pivotal trial outcomes during this period.

**Table 11.** Top sponsors with completed CNS trials, by success rates and positive pivotal trial counts

Sponsor	Negative outcome/primary endpoints not met	Outcome indeterminate	Outcome unknown	Early positive outcome	Positive outcome/primary endpoints met	NA	Total Trials (rank)	Success Rate	Positive Pivotal Trials (count)
AstraZeneca	0	10	5	0	1	4	20 (1)	5.0%	0
Eli Lilly	1	7	4	0	3	3	18 (2)	16.7%	1
Roche	1	3	7	0	6	1	18 (2)	33.3%	2
Johnson & Johnson	2	3	9	0	1	2	17 (3)	5.9%	0
Otsuka Holdings	3	4	1	0	6	2	16 (4)	37.5%	1
Lundbeck	3	3	4	0	2	3	15 (5)	13.3%	1
Boehringer Ingelheim	2	1	4	0	0	5	12 (6)	0.0%	0
Vertex	0	0	2	0	4	6	12 (6)	33.3%	1
AbbVie	0	1	1	0	5	4	11 (7)	45.5%	0
Takeda	1	2	4	0	1	3	11 (7)	9.1%	0
UCB	0	4	0	0	3	4	11 (7)	27.3%	0
Biogen	0	0	5	0	2	2	9 (8)	22.2%	1

Note: Indeterminate designation is assigned to trials when the outcome is neither clearly positive nor negative. Unknown is assigned to trials that have yet to report full results for their primary endpoints. NA indicates trials with no efficacy/safety outcomes to evaluate.

Source: Trialtrive, February 2024

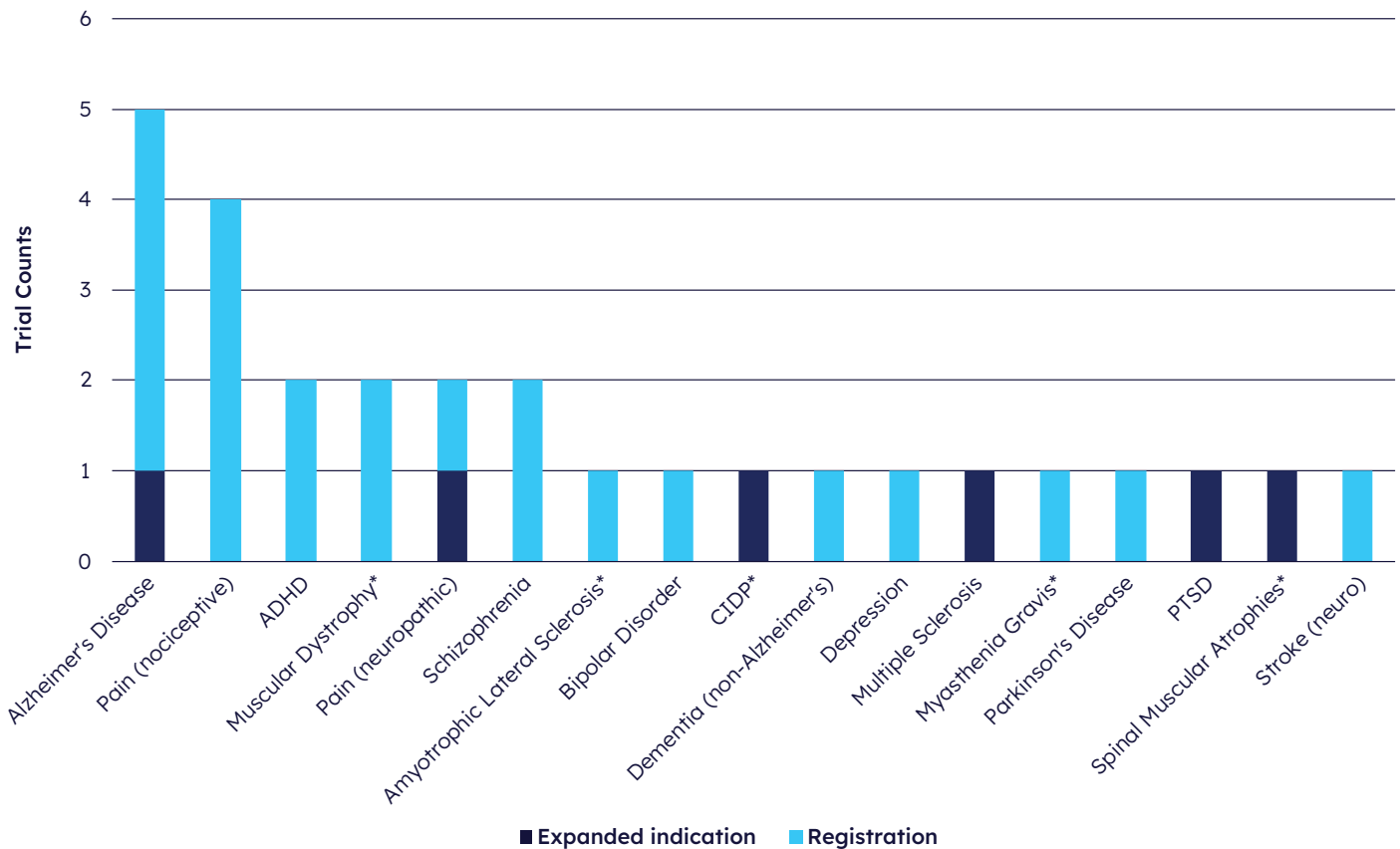


# Annual Completed Clinical Trials Report

The pivotal trials reporting positive outcomes in 2023 focused on 17 of 44 CNS diseases (Figure 5), five of which are rare while others are large indications with high unmet need. Alzheimer’s disease and depression were top diseases for positive outcomes reported (Table

3), and broadly reflected early-phase trials that reported surrogate efficacy endpoints. Registration trials (22) outnumber the expanded indication studies (six) that have historically dominated CNS, as new formulations were developed.

**Figure 5.** Pivotal CNS trials achieving primary endpoints, by disease and filing type



\*Rare disease

ADHD = attention deficit hyperactivity disorder; CIDP = chronic inflammatory demyelinating polyneuropathy; PTSD = post-traumatic stress disorder

Source: Trialtrave, February 2024

# Annual Completed Clinical Trials Report

This analysis identified the pipeline status drugs from positive pivotal CNS studies and assigned them as novel or not, based on whether theirs is a previously proven MOA (Table 12). In contrast with the other top TAs, no pipeline status CNS drugs featured here possess a novel MOA. The “me-too” drugs comprise therapies for a broad spectrum of diseases, such as several successful

Alzheimer’s disease drugs that continue to target beta amyloid antagonist after many years of evaluation. Reformulations continue to play a prominent role in CNS drug development as better management of dosing is sought. For example, new modified-release formulations in Parkinson’s disease, pain, ADHD, PTSD, and schizophrenia were successful during 2023.

**Table 12.** Pipeline status drugs in successful pivotal CNS trials, by sponsor, disease, MOA, and MOA novelty

Drug name	Sponsor	Disease	Mechanism of action	Novel
suzetrigine	Vertex Pharma	Pain (nociceptive)	Sodium channel antagonist	No
centanafadine SR	Otsuka Holdings	ADHD	SNDR inhibitor	No
cyclobenzaprine, TONIX	TONIX Pharma	Pain (neuropathic)	Alpha 1 AR antagonist; Serotonin 2A R antagonist; Histamine H1 R antagonist	No
batoclimab	Harbour BioMed	Myasthenia Gravis*	Fc fragment of IgG R and transporter antagonist	No
HE-3286	BioVie	Alzheimer’s Disease	MAPK-1/3 inhibitor; NF-kappaB inhibitor	No
dextromethadone, Relmada Therapeutics	Relmada Thera	Depression	NMDA R antagonist	No
ciprofloxacin + celecoxib, NeuroSense Thera	NeuroSense Thera	Amyotrophic Lateral Sclerosis*	COX-2 inhibitor; DNA topo II/IV inhibitor	No
tropium chloride + xanomeline, Karuna Thera	Karuna Thera	Schizophrenia	Muscarinic R antagonist; Muscarinic M1 R agonist	No
donanemab	Eli Lilly	Alzheimer’s Disease	Beta amyloid protein antagonist	No
dexamethasone, Oculis	Oculis	Pain (nociceptive)	Glucocorticoid agonist	No
sertraline hydrochloride brexpiprazole	Otsuka Holdings	PTSD	Serotonin modulator; Dopamine modulator	No
vesleteplirsen	Sarepta Thera	Muscular Dystrophy*	Dystrophin stimulant	No

# Annual Completed Clinical Trials Report

dexamethasone sodium phosphate, Taiwan Liposome Company	Taiwan Liposome Company	Pain (nociceptive)	Glucocorticoid agonist	No
carbidopa + levodopa, NeuroDerm	Mitsubishi Tanabe Pharma	Parkinson's Disease	Dopamine R agonist; DOPA decarboxylase inhibitor	No
deuterium-modified dextromethorphan, quinidine, Otsuka Holdings	Otsuka Holdings	Alzheimer's Disease	Neuronal nicotinic R antagonist; NMDA R antagonist; SNRI; Opioid delta R /Sigma 1 R agonist	No

\*Rare disease

Gray fill: China-only

AR = adrenoceptor; NMDA = N-methyl-D-aspartate; R = receptor; SNDR inhibitor = serotonin-norepinephrine-dopamine reuptake inhibitor

Source: Trialtrave and Pharmaprojects, February 2024

## Key Takeaways

The growth of completed trials activity in 2023 reflects broad increases across most TAs, and a clear break from pandemic period fluctuations observed since 2020. The focus on ID and vaccines has tapered and disruptions have abated for other disease trials. The similarities between 2019 and 2023 trends (Figure 1) point to an end to the dominance of pandemic-driven factors. CNS returned to the top three TAs, and the top 20 pharma players continue to dominate global activity, even as geographically focused AOP trials continue to play important roles, particularly for China, as highlighted in the pipeline status drug review for the top three TAs of oncology, A/I, and CNS.

In addition to broad-based completed trials growth in 2023, trial success rates improved

year-over-year, which is another reflection of how the challenges of the pandemic years are no longer driving trends in 2023. These improved outcomes were observed broadly at the disease level as the most active diseases improved success rates from 31.6% to 33.3% (Table 3). Higher success rates also are observed at the top sponsor level, where the average success rate across the most active sponsors increased from 24.7% to 31.3% (Table 6). At the more granular level, the top oncology and A/I sponsors increased their average success rates by 38.9% vs. 28.8% and 28% vs. 20.8%, respectively.

These 2023 trends illustrate that the health and vitality of industry-sponsored clinical activity have been restored.

## About the Author

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Laura has over 30 years of experience within the pharma industry and academia, and currently supports clinical trial intelligence for the autoimmune/inflammation, CNS, and ophthalmology therapeutic areas at Citeline. She has contributed to many Citeline thought leadership publications, including the annual completed trials analysis since 2017. Prior to joining Citeline in 2006, Laura performed research focused on immunology, hepatitis B, and gene regulation.





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