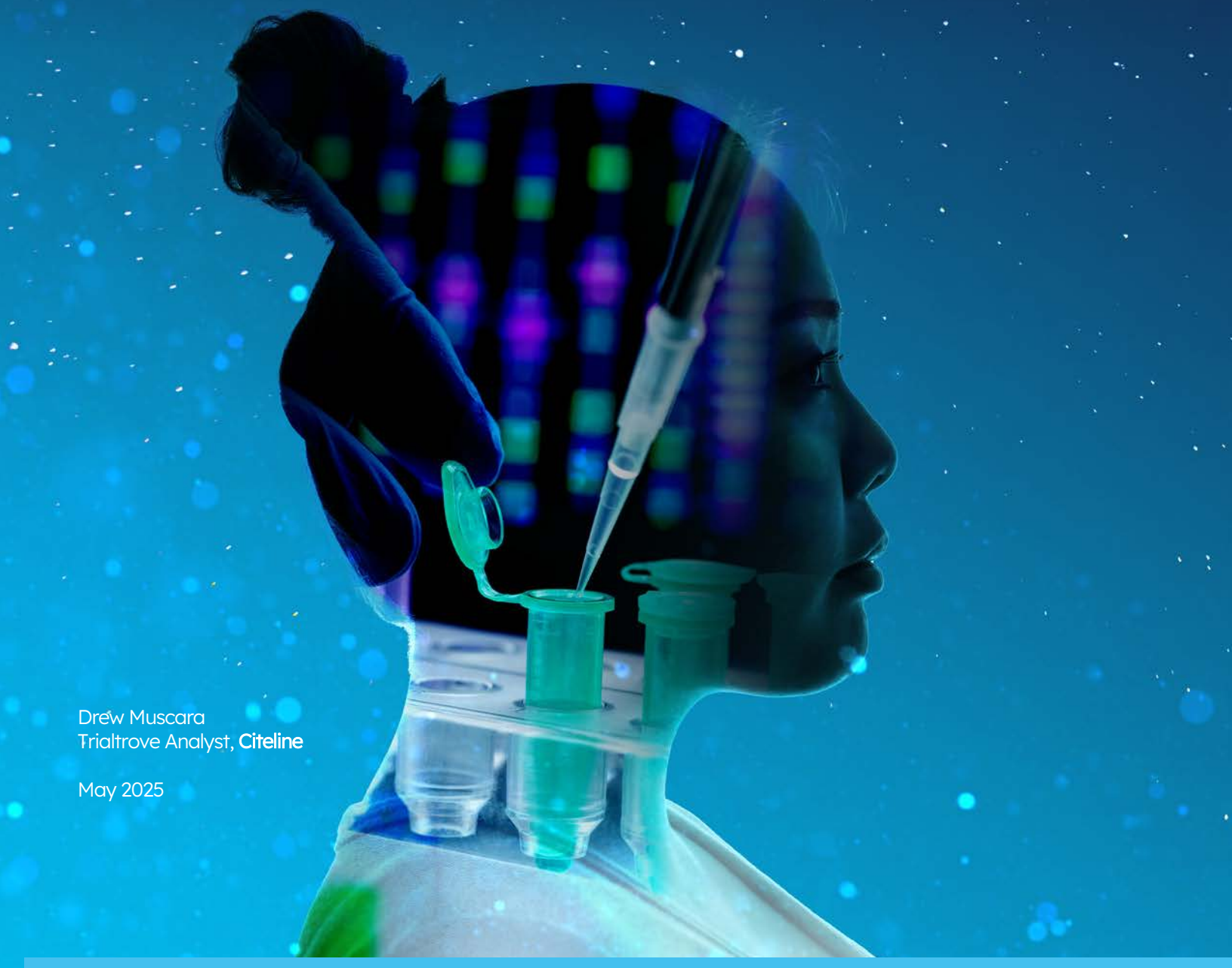
CITELINE | A NEW ERA OF CLINICAL INSIGHT

White Paper

Annual Completed Clinical Trials Report

Key insights and results from our annual review of trends in clinical trials completed in 2024, based on Trialtrove data



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Introduction

Trialtrove recorded 4,903 industry-sponsored clinical studies from Phase I through Phase III/IV that either completed or attained primary endpoints during 2024. This is a 14.2% increase from the previous year that reported 4,295 completed trials evaluated from 2023, representing the greatest increase in the last eight years (2017–2023). An additional 1,153 trials were noted as terminated in 2024, marking an 11.9% rise from those recorded in 2023 (1,030). Following the prior year's increase in completed trials, there is a consecutive rise in the number of studies that reached completed status. As the volume of studies continually compounds, a deviation from the pattern of alternating trial count increases, in 2021 and 2023, and decreases, in 2020 and 2022, is evident. [1]

For the second year in a row, all therapeutic areas (TAs) exhibited greater trial completions than the year before. Although higher completed trial levels were found, vaccines (infectious disease) studies had insubstantial growth compared with other TAs such as oncology and central nervous system (CNS) which saw the most prominent surge in completed trials, respectively. In the post-pandemic landscape, the minimal number of completed vaccine studies marks a stabilization in COVID-19 related programs.

The high performance of the top 20 pharma companies continues to show through the topline trial evaluations. However, with a significant number of completed studies recorded, all other pharma (AOP) have also responded and bolstered their trial influence. As the analysis unfolds, several trends emerge, not only highlighting key disease areas, but also offering new perspectives on novel therapeutics, noteworthy contributors, and much more throughout the course of this white paper.



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Topline Trial Landscape Metrics

The findings from the past year, for each of the TAs, are emphasized within Table 1 and portray the TA ranking along with corresponding trial counts, comparatively. Oncology once again has far outstripped the other TAs and maintained its first-place rank for completed trial activity in 2024. As the distribution of trials tallied for the respective therapy areas mirrors that of 2023, commonality can also be seen with autoimmune/inflammation (A/I) in second rank, closely surpassing CNS, in the third rank. A deeper examination reveals that these top three areas have reached record highs in the total sum of trials completed this last year.

Despite the successive, similar pattern observed across rank order for alternate TAs, modest growth was discerned for each of the six remaining TAs. Although competition over the fourth-place rank was noted between metabolic/endocrinology (met/endo) and infectious disease (ID) in the 2023 results, a stark difference for 2024 shows that met/endo has risen above ID to separate themselves into the appropriate fourth- and fifth-place ranks. The distance placed between met/endo and ID was largely driven by the higher number of completed trials found for the disease of obesity (125). Cardiovascular (CV) has also displayed improvement in trial completions for another year in a row, breaking free from the declines disclosed from 2021 to 2022. Vaccines continue to hold the seventh place, with minimal advancement year over year as the industry moves further into a post-pandemic environment and COVID-19 focused studies wane. Completed ophthalmology and genitourinary (GU) trials remain consistent for rank, but with consideration to volume, ophthalmology's upward trend continues, and new heights reached with 146 trials.

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

Therapeutic area	Ranking				Trial Count[a]			
	2024	2023	2022	2021	2024	2023	2022	2021
Oncology	1	1	1	1	1,466	1,197	1,096	1,222
Autoimmune/Inflammation	2	2	2	3	828	758	669	700
CNS	3	3	4	4	811	678	619	620
Metabolic/Endocrinology	4	4	5	5	698	649	570	545
Infectious Disease[b]	5	5	3	2	672	648	643	726
Cardiovascular	6	6	6	6	377	358	293	303
Vaccines	7	7	7	7	288	286	255	231
Ophthalmology	8	8	8	8	146	136	121	97
Genitourinary	9	9	9	9	70	62	58	62

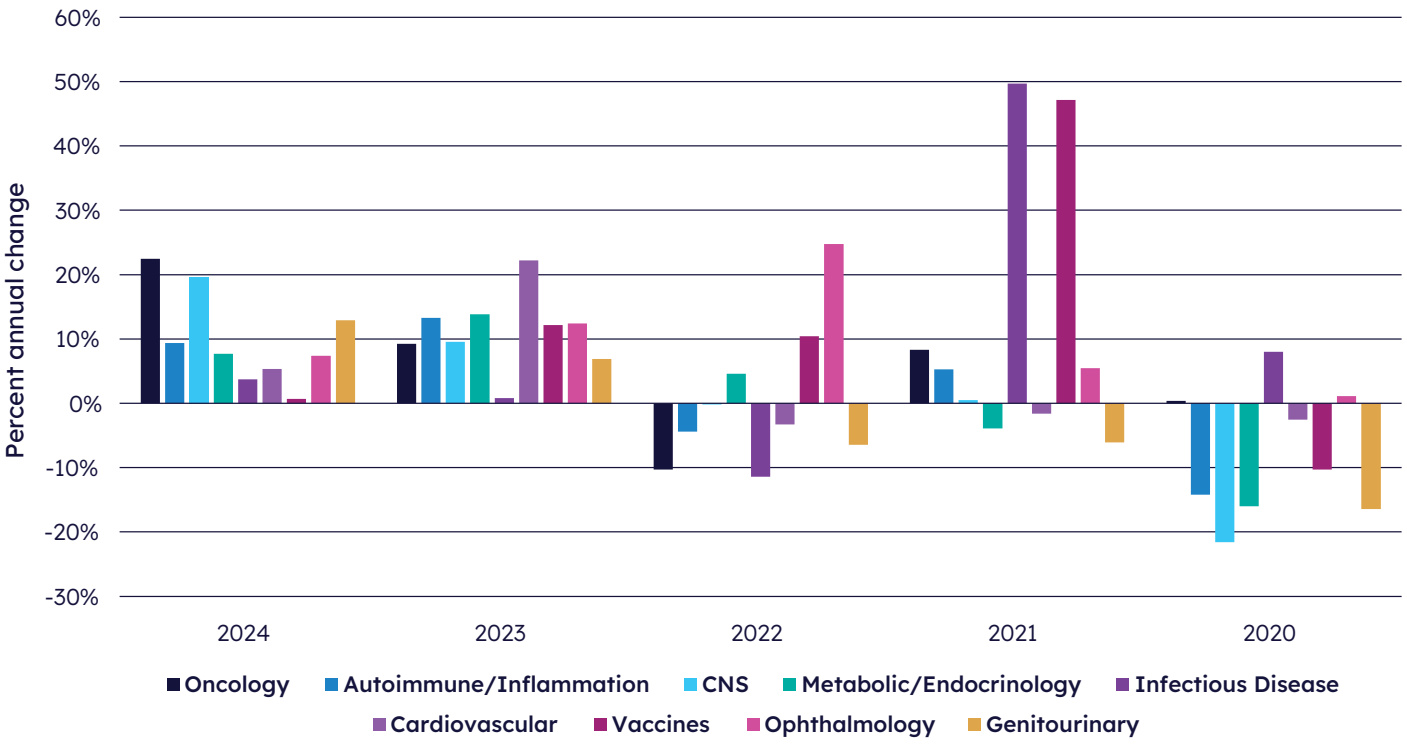
[a]Trials may span multiple therapeutic areas
[b]Excludes Vaccines trials

Source: Trialtrove, February 2025

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In review of the annual changes in trial activity, from 2020 to 2024 (Figure 1), movement was recognized across each TA; however, significant contribution to these values was localized in three TAs. In the last five years, the industry has felt the turbulence of yearly trial increases and decreases with this year being no exception. The TAs which saw the most significant development were oncology (22.5%), CNS (19.6%), and GU (12.9%). Regardless of the previous elevation listed in completed CV trials, there was a lack of advancement outlined through this year's results. Moreover, vaccines was met with little change as the number of studies differed minimally and disclosed a meager 0.7% increase. The percentage growth offers new insights and potential noteworthy disease areas for expansion.

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



Source: Trialtrove, February 2025



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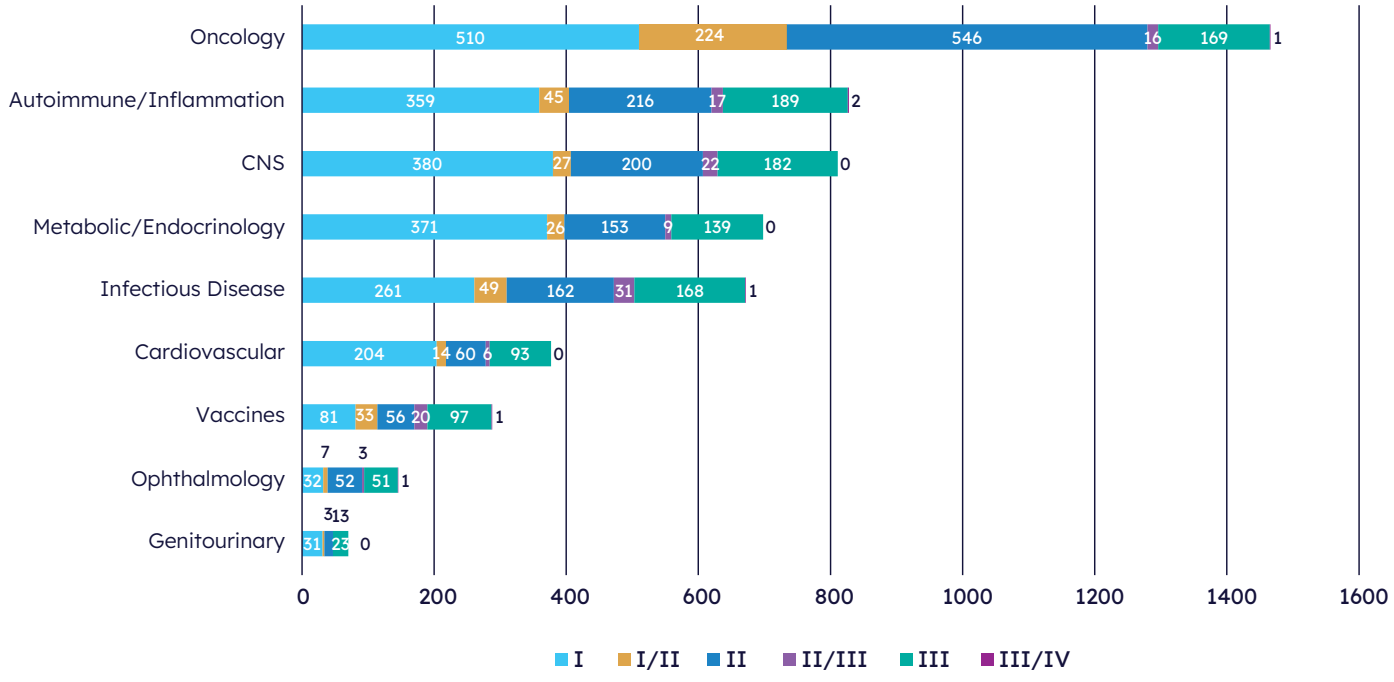
Distribution of 2024 completed trials by therapeutic area and phase

Following in the footsteps of our previous analyses, the distribution of trial counts by phase have matched the format for lower aggregations of late–phase, completed trials (Phase II/III–III, III/IV) and are dominated by the vast majority of early phase, I–II studies (Figure 2). Historically, this has been understood and attributed to many of the stringent requirements imposed for late-stage clinical trials. As specific attention is paid to the distribution of phases, over the last three years, there is a clear relationship in the amount of Phase I/II oncology (15.3%) and vaccine (11.5%) trial completions. Having shown elevated, above-average values, oncology and vaccine TA studies persistently maintain high proportions of Phase I/II trial completions in review of the 2024 counts. Id also made modest movement in Phase I/II study numbers, with consideration to all other TAs, as it reflected a value of 7.3%.

Each of the other TAs provided less variability in range for Phase I/II trials, with an average calculation of 4.2% (~3%–5%). In addition, oncology Phase III percentages remain low (11.5%) compared to the other eight TAs, which averaged greater than twofold higher in Phase III completion (24%).

Minimal noteworthy decline was evaluated during this period as the only phase and TA combinations experiencing drops were Phase II A/I (216 vs. 236), Phase I/II met/endo (26 vs. 29), ID Phase I/II and II/III (49 vs. 52; 31 vs. 32), Phase II CV (60 vs. 65), vaccine Phase II/III (20 vs. 21), ophthalmology Phase I/II and II/III (seven vs. 15; three vs. five), and genitourinary Phase III (23 vs. 30). Despite the distribution similarity and minute drops in trial counts, broad-based growth was experienced across all TAs. The rise in earlier phase trials promotes a promising outlook for several new therapies to advance.

Figure 2. Distribution of industry-sponsored trials completed in 2024, by therapy area and phase



Source: Trialtrove, February 2025

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Top reported trial diseases

The top 10 diseases for trial completions in 2024 (Table 2) have displayed drastic shifts in noteworthy diseases, introductions to rare cancers, and returns of familiar players. This year the top 10 diseases for trial completions were comprised of non-small cell lung cancer (NSCLC), breast cancer, type 2 diabetes (T2D), COVID-19, colorectal cancer, respiratory vaccines, non-Hodgkin's lymphoma (NHL), head/neck cancer, obesity, and pancreatic cancer. Returning to the No. 1 rank for the first time since 2020, NSCLC has reached its highest number of trials (242) in the history of our analyses. Additionally, breast cancer underwent a considerable uptick in completed trials, moving from fifth to second. T2D showed no change in the number of completed trials but has decreased standing to fall into third.

Interestingly, COVID-19 has toppled from first place, after holding this spot for more than three consecutive years and is now seated in fourth. Continuing in the trend of lower completed trial counts, COVID-19 experienced a reduction of 59 completed trials. Furthermore, where COVID-19 vaccine studies are included, these overlapped with respiratory vaccines, and with the steady decline of such studies, no doubt they accounted for the movement of respiratory vaccines into sixth place.

Among the top five, colorectal cancer emerges after narrowly missing the mark in past years. The bottom four ranks are composed of three rare oncology indications (NHL, head/neck cancer, and pancreatic cancer, respectively) and obesity. The greatest upheaval was observed from pancreatic cancer as completed trial quantities leapt from 71 to 124, a 75% jump within the last year.

Several companies also made significant contributions to studies treating a variety of these top 10 diseases. A comparison of noteworthy contributors, cross-referenced from *10 Clinical Trials of 2024,*[2] reveals several key players for met/endo and oncology diseases. These two TAs composed 80% of the top 10 disease areas and buttressed both the trial counts and associated ranking for these indications.



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Among the sponsors supporting these disease indications were Eli Lilly, Amgen, Novo Nordisk, Summit/Akeso, and many more. In the supplemental report of 10 trials, the focus of many of the studies was aimed at subjects with T2D, obesity, and related comorbid conditions. Where Eli Lilly's lead product, tirzepatide, saw success in many of the obesity-related trials, other anti-obesity candidates like Amgen's maridebart cafraglutide and Novo Nordisk's cagrilintide/semaglutide did not find the same overwhelming resolve. However, these mixed reviews still provided meaningful results as the development continues, despite observing reduced efficacy and safety profiles for such products.

Separately, Summit/Akeso's drug candidate (ivonescimab) for head and neck, breast, and colorectal cancers also hit the targets during their Phase II trial. The positive outcomes disclosed from the trial displayed strong efficacy and may prove ivonescimab to be an alternative therapy for Merck & Co.'s Keytruda.

The involvement of met/endo and oncology diseases in the supplemental analysis has further justified the rankings disclosed in Table 2.

Table 2. Top 10 diseases for trials completed in 2024 and comparison to prior three years

Disease	2024 (rank)	2023 (rank)	2022 (rank)	2021 (rank)
Non-small cell lung cancer	242 (1)	202 (3)	160 (4)	207 (2)
Breast cancer	231 (2)	155 (5)	174 (3)	164 (4)
Type 2 diabetes	205 (3)	205 (2)	182 (2)	155 (5)
Novel coronavirus (2019-nCoV, COVID-19)	196 (4)	255 (1)	304 (1)	354 (1)
Colorectal cancer	149 (5)	101 (7)	102 (7)	132 (6)
Respiratory vaccines	137 (6)	162(4)	160 (5)	121 (7)
Non-Hodgkin's lymphoma*	134 (7)	119 (6)	115 (6)	139 (5)
Head/neck cancer*	125 (8)	88 (11)	85 (12)	99 (10)
Obesity	125 (9)	88 (12)	54 (30)	34 (63)
Pancreatic cancer*	124 (10)	71 (24)	54 (31)	72 (21)

*Rare disease

Source: Trialtrove, February 2025



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Trial success rates

Completed trials are assigned an outcome evaluation (positive, negative, unknown, or indeterminate) whether those studies publicly disclosed safety, efficacy, or biomarker/surrogate efficacy outcomes. The success rates of each disease were assessed and categorized based upon those that exhibited the greatest proportion of positive outcomes and were ranked based on the success of total completed trials in 2024 (Table 3).

Table 3. Diseases with ≥25 completed trials attaining primary endpoint

Disease	I	I/II	II	II/III	III	III/IV	Total	% of all Trials	Rank #
Respiratory vaccines	12	7	12	6	29	0	66	48.2%	1
Novel coronavirus (2019-nCoV, COVID-19)	20	7	22	5	20	0	74	37.8%	2
Obesity	20	1	16	0	10	0	47	37.6%	3
Multiple myeloma*	10	2	10	0	7	0	29	36.3%	4
Bladder cancer	9	2	12	0	3	0	26	34.7%	5
Breast cancer	23	13	30	2	11	1	80	34.6%	6
Renal disease	11	0	10	0	6	0	27	34.2%	7
Dyslipidemia	10	1	4	0	13	0	28	32.9%	8
Non-Hodgkin's lymphoma*	13	4	22	1	4	0	44	32.8%	9
Psoriasis	9	2	7	0	12	0	30	32.6%	10
Soft tissue sarcoma*	8	4	13	0	2	0	27	32.5%	11
Esophageal cancer*	12	7	14	0	3	0	36	30.5%	12
Non-small cell lung cancer	24	7	27	0	15	0	73	30.2%	13
Neuroendocrine cancer*	10	2	10	0	5	0	27	30.0%	14
Type 2 diabetes	15	1	15	0	30	0	61	29.8%	15
Prostate cancer	10	3	14	0	5	0	32	29.4%	16
Pancreatic cancer*	15	10	9	0	1	0	35	28.2%	17
Gastric cancer	14	6	11	0	2	0	33	28.2%	18
Head/neck cancer*	9	6	15	0	4	0	34	27.2%	19
Colorectal cancer	17	5	17	1	0	0	40	26.8%	20
Ovarian cancer*	14	6	6	0	5	0	31	26.7%	21
Nociceptive pain	7	1	5	2	10	0	25	26.6%	22
Alzheimer's disease	15	2	3	2	3	0	25	25.5%	23
Atopic dermatitis	5	3	6	1	10	1	26	24.5%	24
Melanoma	12	7	5	0	3	0	27	23.1%	25

*Rare disease
Rank based on percentage of trials attaining primary outcome per disease

Source: Trialtrove, February 2025

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A significant rise in the number of diseases achieving 25 or more successful trials was observed throughout the 2024 results. The total value of diseases nearly doubled that which was returned from 2023 (25 vs. 13). Cross-referencing the indications portrayed in the top 10 diseases for completed trials (Table 2), each was also classified as a high-performing and successful disease area containing 25 or more positive, completed trials. The diseases reflected were heavily influenced by the large number of oncology trial completions listed for the previous year.

Our analysis shows that more than half the diseases outlined (15 of 25) were comprised of oncological indications. This comes as little to no surprise with the immense level of trial activity recorded for the respective TA. Lesser representation has been noted for many other TAs including ID (one), vaccines (one), CV (one), AI (two), CNS (two), and met/endo (three). Not unlike historical reports, GU and ophthalmology diseases did not meet the minimum 25-trial requirement to sufficiently emphasize successful results.

Topping the chart, breast cancer achieved a definitive number of successful trials (80) and earned a sixth-place rank in terms of overall success rate (34.6%). Following on, respiratory vaccines indicated meaningful progression with 66 completed trials attaining primary endpoints, five more than that which was found for preceding trials in 2023 (61). The respiratory vaccine trial growth was also met with profound success as it returned to first place after a year hiatus and boasted a 48.2% success rate for trials in 2024. Much of the accomplishment in respiratory vaccines can be attributed to the support of COVID-19, which was not far behind and continues to display lower annual completed trial counts. COVID-19 studies have once again decreased from 79 to 74, but have fared well for trial success, yielding 37.8% and moving from 11th rank up to second. The evaluation of these diseases also reveals that 40 of the 66 successful respiratory vaccine studies were COVID-19 vaccine trials. NHL continued to show an upward trajectory and reported more positive trials, 44 vs. 38, and advancing to ninth from 10th in 2023.

Alternate diseases that saw optimistic movement in the number of positive trials were obesity, ranking third; multiple myeloma, ranking fourth; and bladder cancer, ranking fifth. The count of obesity trials, with favorable outcomes not only increased to 47 from 29 but also the percentage of success was noteworthy as it climbed to 37.6% after the previous reported value of 33.0%. These triumphs came as positive Phase I obesity trials saw a twofold improvement year over year. Returning to the top diseases and trials attaining primary endpoints, multiple myeloma studies have not met these criteria since 2019. However, multiple myeloma had 29 positive trial completions which led to its fourth-place designation. Ranking fifth and presenting a modest 34.7% success rate, bladder cancer returns after not being included in this analysis for four years (since 2020).

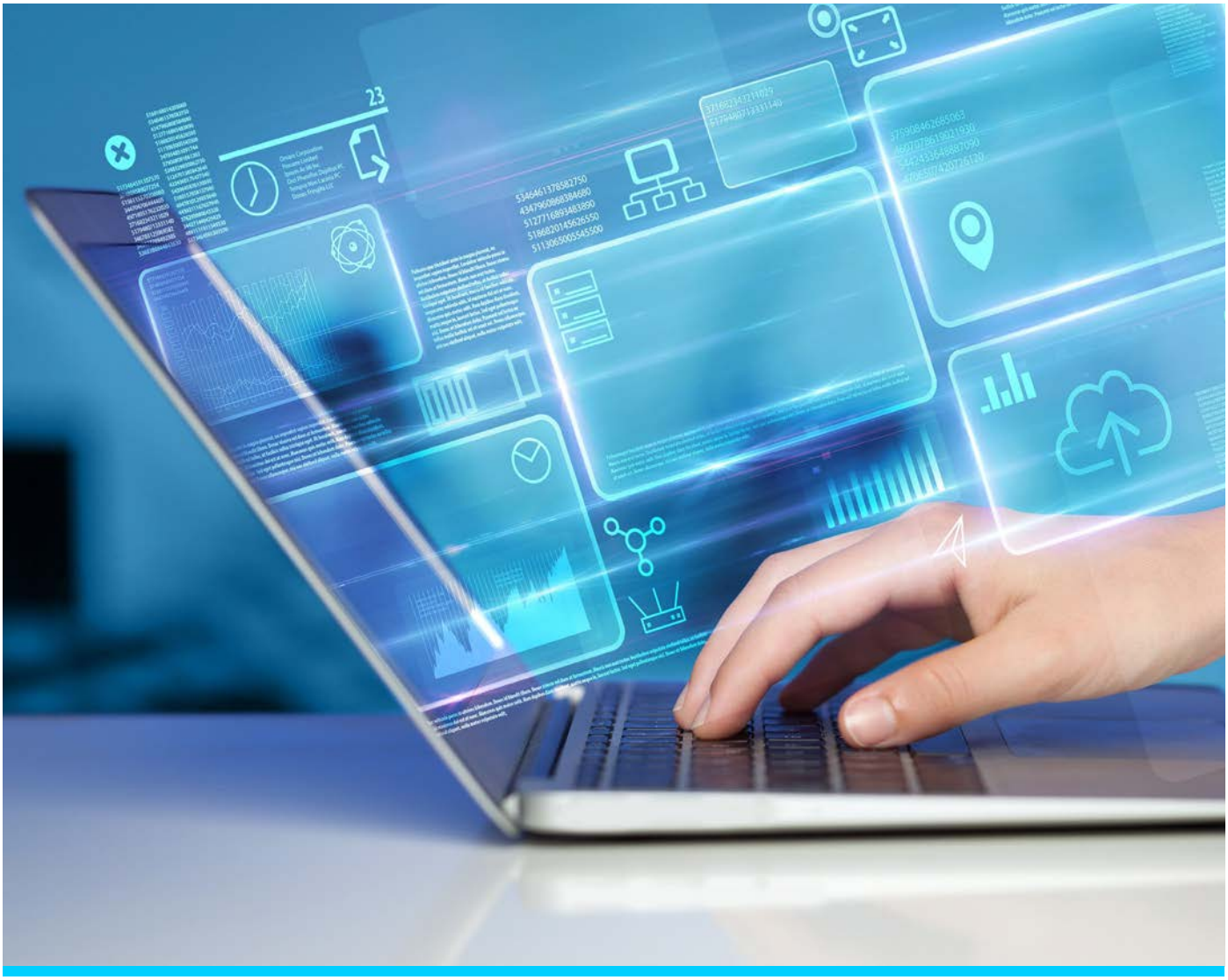
Separately, Alzheimer's disease, atopic dermatitis, colorectal cancer, T2D, NSCLC, and dyslipidemia saw regression in their number of positive trials, percent of success, and subsequent ranks. The most drastic change was observed for Alzheimer's disease, which sank to 23rd after being poised at first in 2023, with successful trial percentages and counts dropping from 39.7% to 25.5% and 31 to 25, correspondingly. Also suffering steep decline, the percentage of atopic dermatitis trials with advantageous results markedly moved from 34.1% to 24.5% as positive trials also shifted from 31 to 26 in a year's time. Colorectal cancer (20th vs. 13th), T2D (15th vs. eighth), NSCLC (13th vs. ninth), and dyslipidemia (eighth vs. sixth) had less severe differences in success rate and rank, albeit still reflecting noticeable change over the year before.

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Two indications (depression and liver cancer) did not achieve ≥25 positive trials and were not evaluated in 2024.

Several substitutes further contributed to the wide array of diseases found in the results, consisting of renal disease (27 trials; rank seven), psoriasis (30 trials; rank 10), soft tissue sarcoma (27 trials; rank 11), esophageal cancer (36 trials; rank 12), neuroendocrine cancer (27 trials; rank 14), prostate cancer (32 trials; rank 16), pancreatic cancer (35 trials; rank 17), gastric cancer (33 trials; rank 18), head/neck cancer (34 trials; rank 19), ovarian cancer (31 trials; rank 21), nociceptive pain (25 trials; rank 22), and melanoma (27 trials; rank 25).

The average, overall success rate for diseases maintaining 25 or greater positive trial results was calculated at 31.2% and contrasted with the 33.3% success value noted in 2023. In comparison, the rates evaluated from the top 11 ranking diseases (48.2%–32.5%), exceeded the overall average and highlighted the success of many promising drugs for these disease areas.



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Leading Trial Sponsors

Steering attention toward top sponsors involved in completed trials, those with the most trial activity and success have been identified for 2024. In our analysis, the results focus on trials of both top 20 pharma, evaluated based upon sales data in the annual In Vivo Outlook 2025 dataset,[3] in conjunction with a separate group comprised of AOP, excluding generics-only companies. The top five sponsors by overall completed trial counts in 2024 remain representative of companies listed within the top 20 pharma (Table 4). This year the annual analysis shows that not only did Merck & Co. hit a new pinnacle for the number of completed trials but also has moved into the top rank. Merck & Co.'s record totals have progressed steadily since 2022, with nearly twice the completed trial amounts. This is the first year since the inception of our Annual Completed Trials report that Merck & Co. has claimed the title of first rank. Additionally, in review of the studies sponsored by Merck & Co., more than half (128; data not shown) were inclusive of oncological indications. Tracking closely behind, Pfizer has also shown decisive progression in trial volume as it stepped out of first and into second place. Following suit, AstraZeneca (AZ) returns to third place after steadily maintaining its second ranking for three years. Bristol Myers Squibb (BMS) had continuity in fourth rank as it remains unchanged throughout the 2024 results. At the bottom of the top five, showing notable improvement, Roche completed 34 more studies in 2024, validating its standing at fifth place.

Table 4. Top five sponsors* completing trials in 2020–2024

Sponsor	2024 (rank)	2023 (rank)	2022 (rank)	2021 (rank)	2020 (rank)
Merck & Co.	228 (1)	152 (3)	116 (4)	140 (3)	127 (6)
Pfizer	218 (2)	174 (1)	165 (1)	134 (4)	152 (2)
AstraZeneca	192 (3)	170 (2)	159 (2)	175 (2)	142 (3)
Bristol Myers Squibb	184 (4)	147 (4)	142 (3)	177 (1)	139 (4)
Roche	152 (5)	118 (6)	116 (4)	115 (7)	138 (5)

*Trial count includes cosponsored trials

Source: Trialtrove, February 2024

Expanding the scope of the analysis, a supplemental list of sponsors that completed 50 or more trials shows the following sponsors and related counts: Eli Lilly (111), Novartis and Boehringer Ingelheim (BI; 101), GlaxoSmithKline (GSK; 100), Johnson & Johnson (J&J; 99), Sanofi (80), Jiangsu Hengrui Pharma and AbbVie (75), Takeda (68), Novo Nordisk (63), and Amgen (61). Furthermore, the other top 20 sponsors that did not report completing 50 or more trials were Gilead, Bayer, Teva, Viatris, and CSL.

In retrospect, the top 20 pharma completed a total of 1,953 trials, over 25% more trials than disclosed the year before (1,561 trials).

Through this assessment, a pattern of decreases in the top 20 pharma participation rates changed with a 2% uptick (38% vs. 36%).[1] In comparison, AOP counts also saw a rise in number and contrasted with top 20, percentages were found to be 38% vs. 69%.

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Success of industry sponsors was measured by their overall counts of positive trials that obtained primary endpoints and was dispersed by trial phase in Table 5. The number of sponsors with 25 or more positive trials has slightly diminished in the last year, as the count was rendered to 10 sponsors from last year's values, which reflected 11 sponsors meeting these criteria. In terms of those included in the list, little change was shown for the sponsors observed; however, substitutes were found where Sanofi (22) and AbbVie (20) did not report greater than 25 positive trial results, but GSK had (32). Showing consistency in the prominent number of positive trial counts (68), AstraZeneca has again secured itself as the top company attaining trial primary endpoints. Also showing distinct character in the successful performance of completed trials, Merck & Co. has bridged the "second-place" gap as the company advanced from 47 trials, reflected in 2023, to 61 trials in 2024. The top two high-performing sponsors have shown nearly twice as many trials as the bottom four sponsors by these metrics, including Jiangsu Hengrui Pharma (32), GSK (32), Novartis (30), and Eli Lilly (28).

Table 5. Companies attaining primary endpoints in ≥25 trials, by phase (2024)

Sponsor	I	I/II	II	II/III	III	III/IV	Total
AstraZeneca	18	8	26	0	15	1	68
Merck & Co.	7	5	32	2	15	0	61
Bristol Myers Squibb	11	7	18	0	10	0	46
Roche	4	3	22	1	15	0	45
Pfizer	8	6	16	0	9	0	39
Johnson & Johnson	9	2	11	3	13	0	38
Jiangsu Hengrui Pharma	6	2	14	2	8	0	32
GlaxoSmithKline	8	3	4	0	17	0	32
Novartis	4	2	8	0	15	1	30
Eli Lilly	5	1	8	0	14	0	28

Source: Trialtrove, February 2025

Sponsors completing a minimum of 40 trials were further evaluated and the success of corresponding trials was determined by the individual phase and total trial percentages (Table 6). Significant effort was made by each of the companies reported and is seen as a large number of sponsors met these milestones in 2024 (20). The company leading with the greatest percentage (47.6%) was Sino Biopharmaceuticals; its trials were predominantly run in China alone. Conversely, the lowest success rate was observed for Qilu Pharmaceutical Co. (12.2%), whose trials were also conducted exclusively in China.

Once again J&J returned with growth as it reflected a greater than 10% annual yield (38.4%), after last recording of 27.8% success in 2023. In comparing the total trial percentages

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and contrasting the average for all sponsors, with the prior year's data (31.3%),[1] a drop in average success values was calculated (28.8%). However, a clear division was shown where 10 sponsors completed an above-average number of successful trials and equally 10 sponsors fell below this threshold.

Success rates are dependent upon several factors, including the availability of publicly reported trial outcomes and whether the outcome results reflect meaningful evidence of success, by meeting or exceeding the primary endpoint(s). Additionally, Phase I trials without safety or efficacy/surrogate efficacy-related outcomes can further impact the success rates for sponsors. As late-phase trials are used to substantiate approval of study drugs, the elevated level of related trial success rates observed for many of these sponsors may equate to many new therapies reaching the market.

Table 6. Percentage of 2024 trials* attaining primary endpoint, by phase, for companies completing ≥40 trials

Sponsor	I	I/II	II	II/III	III	III/IV	Total
Sino Biopharmaceuticals	19.0%	50.0%	69.2%	0.0%	100.0%	0.0%	47.6%
Jiangsu Hengrui Pharma	16.2%	50.0%	66.7%	66.7%	80.0%	0.0%	42.7%
Johnson & Johnson	42.9%	22.2%	35.5%	100.0%	37.1%	0.0%	38.4%
AstraZeneca	26.5%	47.1%	41.3%	0.0%	36.6%	100.0%	35.4%
Bayer	12.5%	66.7%	16.7%	0.0%	75.0%	0.0%	34.9%
Novo Nordisk	9.5%	100.0%	27.3%	0.0%	50.0%	0.0%	33.3%
GlaxoSmithKline	22.2%	42.9%	17.4%	0.0%	54.8%	0.0%	32.0%
Incyte	14.3%	33.3%	36.4%	0.0%	50.0%	0.0%	30.6%
Novartis	28.6%	20.0%	19.5%	0.0%	44.1%	100.0%	29.7%
Roche	8.9%	20.0%	43.1%	50.0%	38.5%	0.0%	29.6%
Sanofi	13.3%	10.0%	25.0%	100.0%	42.9%	0.0%	27.5%
Merck & Co.	8.9%	19.2%	39.5%	66.7%	38.5%	0.0%	26.8%
AbbVie	21.9%	16.7%	25.0%	0.0%	35.0%	100.0%	26.7%
Eli Lilly	8.6%	25.0%	36.4%	0.0%	56.0%	0.0%	25.2%
Bristol Myers Squibb	28.9%	17.9%	22.5%	0.0%	40.0%	0.0%	25.0%
Amgen	23.8%	0.0%	28.6%	0.0%	26.7%	0.0%	24.6%
Boehringer Ingelheim	13.7%	33.3%	16.7%	0.0%	45.5%	0.0%	17.8%
Pfizer	7.5%	31.6%	27.6%	0.0%	30.0%	0.0%	17.8%
Takeda	11.1%	0.0%	22.2%	50.0%	20.0%	0.0%	17.6%
Qilu Pharmaceutical	3.4%	0.0%	40.0%	100.0%	20.0%	0.0%	12.2%

*Trial count includes cosponsored trials.

Source: Trialtrove, February 2025

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Top 3 Therapeutic Areas: Assessment by Top Sponsors, Diseases with Positive Pivotal Trials, and Pipeline Therapeutics

A granular evaluation of the top-level data illuminated the three highest-performing TAs. Continuing in the trend of our previous reports, oncology, A/I, and CNS have remained in the upper third of completed trial TAs. These three TAs collectively accounted for 58% of the total trial completions and continue to show their preeminence. Separate analyses for the resultant TAs elucidate distinguished sponsors, major and rare diseases, and novel drugs in pipeline.

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

The top 10 sponsors are ranked by total volume of completed clinical trials, categorized by success rates across every trial phase, and displayed with the count of positive pivotal trials (Table 7). Many of the same companies sponsoring oncology trials were maintained through the list, albeit with higher trial values recorded in 2024. Four additions were found in these results: Sino Biopharmaceuticals, Incyte, BeiGene, and Takeda. Three of these are AOP sponsors, apart from Takeda. For the fourth year in a row, BMS showed great achievement as it not only maintained the first position but also increased trial volumes to 145 from 108. In comparison to the number of trials completed by BMS, more than half the sponsors (ranks seven to 10) completed only a quarter of BMS's trial volume. Retaining the second-place ranking with a large number of completed trials, Merck & Co. trailed closely behind. Roche (95) and AZ (94) traded third and fourth places, despite completing a nearly identical number of oncology trials in the past year. Although moving to fourth place, AZ brandished the highest success rate of oncology trials (55.3%). Pfizer remained unchanged in rank, at fifth, but raised the number of completed trials (81 vs. 56) and simultaneously lowered its success rate from 39.3% to 27.2%. Novartis also established a presence with increased trial volumes as it moved from 30 to 41, while advancing two spots into sixth rank. Several sponsors, both top 20 pharma and AOP, contested for rankings seven (Jiangsu Hengrui Pharma and J&J), eight (Sino Biopharmaceuticals and Incyte), and 10 (BeiGene and Takeda), with matching trial counts seen in the appropriate column. Amgen separated itself in ninth as the company sponsored 27 completed oncology studies, one more than that which was returned the year prior (26).

In terms of positive pivotal trials, 10 of the 13 sponsors emphasized positive outcomes for their pivotal trials. Pivotal trials were denoted as registration trials, those seeking initial marketing approval for select therapies, or expanded indication studies, those evaluating treatment for alternate diseases with previously approved drugs. In descending order of positive pivotal trials, AZ led with the highest value (11), followed by Sino Biopharmaceuticals (six), Roche and Pfizer (five), Merck & Co. and J&J and Incyte (four), Novartis (two), and BMS and Takeda (one). In addition, Jiangsu Hengrui Pharma, Amgen, and BeiGene reported no pivotal trial results for this timeframe. As focus is drawn to the positive pivotal results for Sino Biopharmaceuticals, it is important to note that each of these was solely conducted in China.

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In stark comparison to the 2023 trial results (567), the sum of top oncology sponsors' trial counts totaled 777 and derived a 37% increase in trial activity. Nevertheless, the overall trial success rate of sponsors noted in the top 10 diminished four points from 38.3% to 34.2%. The perceived decrease in success rate from 2024 to 2023 came as many of the trials had indeterminate, unknown, or no outcomes altogether (464 vs. 296). While there was a surplus of uncertain trial outcomes, higher quantities of trials also indicated positive results comparatively (266 vs. 217).

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

Sponsor	Early positive outcome	Positive outcome/ primary endpoint(s) met	Outcome indeter-minate	Negative outcome/ primary endpoint(s) not met	Outcome unknown	N/A	Total trials (rank)	Success rate	Positive pivotal trials (count)
Bristol Myers Squibb	0	34	40	17	53	1	145 (1)	23.4%	1
Merck & Co.	0	41	43	7	35	2	128 (2)	32.0%	4
Roche	0	31	18	6	38	2	95 (3)	32.6%	5
AstraZeneca	0	52	15	7	19	1	94 (4)	55.3%	11
Pfizer	0	22	31	4	21	3	81 (5)	27.2%	5
Novartis	0	12	9	2	17	1	41 (6)	29.3%	2
Jiangsu Hengrui Pharma	0	14	1	0	10	4	29 (7)	48.3%	0
Johnson & Johnson	0	12	6	0	11	0	29 (7)	41.4%	4
Sino Biopharma-ceutical	1	14	0	0	10	3	28 (8)	53.6%	6
Incyte	0	9	5	1	12	1	28 (8)	32.1%	4
Amgen	0	7	17	0	3	0	27 (9)	25.9%	0
BeiGene	0	12	4	0	10	0	26 (10)	46.2%	0
Takeda	0	5	6	3	12	0	26 (10)	19.2%	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: Trialtrove, February 2025

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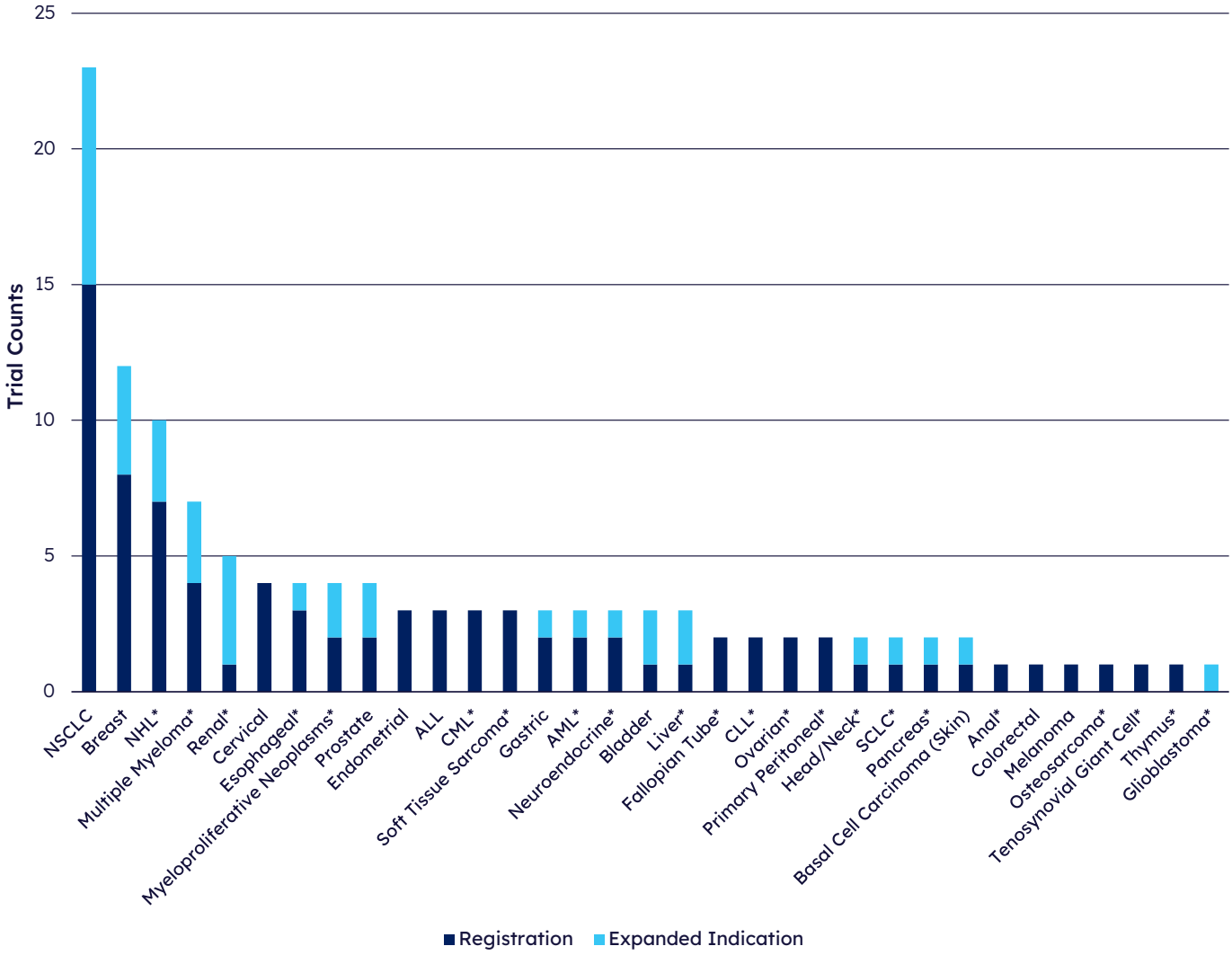
Shifting focus to the disease level measurements, successful pivotal trials encompassed 33 diseases, including 22 denoted as rare. Rare indications are categorized in Trialtrove based on disease prevalence (1 in 2,000 people in the EU or 1 in 1,600 in the US). Note, trials recruiting subjects with a broad range of diseases are tallied by individual disease in the graph (Figure 3). Most trials were tagged as registration (87) with fewer trials being counted as expanded indication (40). The sum of successful pivotal trials by disease (127) has jumped substantially higher than the number recorded for 2023 (83). Comparing the precise number of successful trials referencing pivotal attributes from 2024 to 2023, this number has also moderately risen to 88 from 79. A deeper examination of the indications involved signifies that the top disease with the largest number of both registration and expanded indication tags was once again NSCLC as it reached a new peak, with 23 positive pivotal trials. In the last two years, NSCLC has seen double growth with 11 pivotal trials found in 2022 and 13 in 2023.

Also exhibiting strong advancement, breast cancer positive pivotal trials showed threefold improvement over last year (4) as the current results highlighted 12 trials for the 2024 figures. NHL revealed modest growth in pivotal counts (10) after two consecutive years of stasis (8, 2022; 8, 2023). Other indications that showed expansion were hematological cancers (multiple myeloma, myeloproliferative neoplasms, CML, and AML). The pivotal hematological cancer trials collectively totaled 32 trials which, from our previous report, have almost multiplied by a factor of three (11). Yearly counts were unchanged in several of the diseases, consisting of only solid tumor indications for renal, cervical, esophageal, bladder, liver, fallopian tube, ovarian, tenosynovial giant cell tumor, and primary peritoneal cancer. Additions and returns to the analysis included endometrial, acute lymphocytic leukemia (ALL), soft tissue sarcoma, gastric, head/neck, pancreatic, basal cell carcinoma, anal, osteosarcoma, thymus, and gioblastoma. Contrary to expectation, the large influx of pivotal trials did not reflect a large uptick in trials sponsored by top 20 pharma but rather showed minimal change, differing with two trials as the count moved to 36 from 34 (data not shown), year over year.



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



*Rare disease

Source: Trialtrove, February 2025

Further sifting through the trial details, the study drugs in pipeline status associated with the positive pivotal trials were also analyzed to provide an overview of the developing company, drug name, focus disease(s), key mechanism of action (MOA), and MOA novelty (Table 8). MOA novelty is indicated to reflect whether the corresponding drug's MOA was previously proven. The table below shares that five of these drugs had novel MOAs as determined from their Pharmaprojects profiles.

Three of the five drugs containing novel mechanisms are being developed by companies whose trials exclusively engaged China (gray fill). Sino Biopharmaceuticals contained two novel MOA drugs (culmerciclib & TQB-3525) and were returned for the positive outcomes in their Phase III breast cancer and Phase II NHL trials.

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The separate two drugs with MOA novelty are both targeted for the rare indications of esophageal cancer (Roche) and head/neck cancer (ISA Pharmaceuticals). The Roche candidate, tiragolumab, continues to exhibit positive results as it maintained its position in this report for the successful results in its pivotal Phase III trial, which took place in China, Hong Kong, Singapore, South Korea, Taiwan, and Thailand.

A top-down view of the pipeline status drugs identifies 16 drugs that did not contain experimental MOAs. Excluding GSK's candidate seeking approval for multiple myeloma, each of the additional drugs was developed by AOP and nearly equal representation was shown for drugs involved in China-only trials (7) compared to multinational studies (8). Further, the drugs that did not include new MOAs and instead demonstrated similarity to existing, already-approved drugs would be considered "me-too" pharmaceutical products. Many of the existing MOAs include familiar cyclin-dependent kinase inhibitors, immune-oncology (IO) therapies, T-cell stimulants, apoptosis stimulants, and more. A large portion of the "me-too" drugs mostly likely will support local rather than global regulatory filings and would be used to provide alternatives to the already-marketed 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
culmerciclib	Sino Biopharmaceutical	Breast	CDK 2 inhibitor; CDK 4 inhibitor; CDK 6 inhibitor	Yes
TQB-3525	Sino Biopharmaceutical	NHL*	PI3 kinase alpha inhibitor; PI3 kinase delta inhibitor	Yes
tiragolumab	Roche	Esophageal*	TIGIT R antagonist; IO therapy; Immune checkpoint inhibitor	Yes
peltopepimut-S	ISA Pharmaceuticals	Head/Neck*	Immunostimulant; HPV16 E7 inhibitor; HPV16 E6 inhibitor; IO therapy	Yes
maihuatinib	China Grand Enterprises Pharmaceuticals & Healthcare/Huadong Medicine Co.	NSCLC	ErbB-2 antagonist; EGF R kinase inhibitor	Yes
pimicotinib	Abbisko Therapeutics Co.	Tenosynovial Giant Cell*	CSF-1 receptor antagonist; CSF-1 R TKI; IO therapy	No
BPI-16350	Betta Pharmaceuticals Co.	Breast	CDK 4 inhibitor; CDK 6 inhibitor	No

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pertuzumab, Henlius Biopharma-ceuticals	Shanghai Fosun Pharmaceutical (Group) Co.	Breast	ErbB-2 antagonist; ErbB-2 TKI; IO therapy	No
glecirasib	Beijing Jacobio Pharmaceuticals Co.	NSCLC	K-Ras inhibitor; IO therapy	No
lerociclib	Catalent	Breast	CDK 4 inhibitor; CDK 6 inhibitor	No
imatinib, Inhibikase	Inhibikase	CML*	Abl R TKI; Bcr-Abl inhibitor	No
sevacizumab	Shanghai Xianxiang Medical Technology Co.	Endometrial; Fallopian Tube*; Ovarian*; Primary Peritoneal*	VEGF R antagonist	No
satricabtagene autoleucel	CARsgen Therapeutics	Esophageal*; Gastric; Pancreas*	T cell stimulant; IO therapy; Claudin 18 antagonist	No
pulkilumab injection	Chongqing Precision Biotech Co.	ALL	T cell stimulant; IO therapy	No
abexinostat	Rottendorf Pharma	NHL*	HDAC inhibitor; Apoptosis stimulant; Cell cycle inhibitor	No
belantamab mafodotin	GlaxoSmithKline	Multiple Myeloma*	TNF receptor antagonist; B-cell maturation antigen antagonist; Tubulin polymerization inhibitor	No
ziftomenib	Kura Oncology	AML*	Menin-MLL inhibitor	No
lutetium (177Lu) edotreotide	ITM Solucin	Neuroendocrine*	Somatostatin R 2 agonist; Radioemitter, beta	No
Nidlegy	Philogen	Melanoma	Interleukin 2 R agonist; TNF R agonist	No
iopofosine	Cellectar Biosciences	CLL*; NHL*; Multiple myeloma*	DNA inhibitor; Radioemitter, beta; Radioemitter, gamma	No
aglatimagene besadenovec	Candel Therapeutics	Prostate	DNA directed DNA polymerase inhibitor; TK stimulant; Immunostimulant	No

*Rare disease
Gray fill: China-only trial

Source: Trialtrove and Pharmaprojects, February 2025

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

The top sponsors for A/I were similarly ranked and success established by quantifying the total and positive pivotal trial counts along with calculated success rates (Table 9). The analysis contains 12 total sponsors, each of which was included in last year's analysis and has returned with varying rank and trial counts. Many of the top sponsors are classified as "top 20," with the exception of Incyte, which has maintained involvement in the top sponsor report and was the only AOP sponsor evaluated. Although many familiar sponsors were identified this year, a significant amount of shuffling occurred with respect to previous rankings. In fact, the only sponsor which had no change in rank (fifth) was J&J.

Noteworthy, positive movement of ranking and trial totals were observed for first place with AZ (40 vs. 20), GSK in third (27 vs. 10), Pfizer in fourth (25 vs. 23), and Incyte in seventh (21 vs. 16). The rapid growth experienced by AZ and GSK allowed each to jump seven places from seventh to first and 10th to third, respectively.

Contrasting these positive results, downward progression was noted for the remaining seven sponsors. Eli Lilly dropped out of first and into second but secured higher trial counts (30 vs. 28) and greater success rates (23.3% vs. 17.9%). Juxtaposed to this, BI also shifted from second to third, showing no annual change in trial volume or success rates and matching 27 trials and a 14.8% success metric for another year. Sanofi moved from fourth to sixth, showing evidence of lower trial numbers (22 vs. 24) and a less-optimistic percentage of success (31.8% vs. 41.7%). Similarly, BMS moved from third to seventh with 21 vs. 26 trials completed. Novartis also lost standing as it fell from sixth to eighth and completed one less trial than the prior year (20 vs. 21). Following, AbbVie was knocked from third to ninth, recounting 18 trials in 2024 compared to 26 in 2023 and reducing its success rate in half (16.7% vs. 34.6%). Rounding out the list, Amgen had minor displacement moving one spot from ninth to 10th and signifying lower percentages of success with 23.5% vs. the previously recorded value of 42.9%.

The trial totals for top A/I sponsors summed to 291 and yielded a 6% increase over the 2023 results (274). A slight regression in the average trial success rate for these companies was understood as the value changes from 29.1% to 25.8%, presently. The trial totals across each of the top sponsored studies were also consistent with a lower average success rate as the number of trials without definitively positive outcomes (negative, indeterminate, unknown, or NA) changed from 200 to 227. These higher non-positive outcomes were the primary catalyst for the determination of lower success values noted in these completed A/I studies. Conversely, positive pivotal trials underwent a slight increase, equaling the sum of 19 reported by top sponsors. In an evaluation of these measures, J&J was found to have the greatest number of pivotal trials including one expanded indication and four registration trials.



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

Sponsor	Early positive outcome	Positive outcome/ primary endpoint(s) met	Outcome indeter-minate	Negative outcome/ primary endpoint(s) not met	Outcome unknown	N/A	Total trials (rank)	Success rate	Positive pivotal trials (count)
AstraZeneca	0	10	1	3	18	8	40 (1)	25.0%	0
Eli Lilly	0	7	3	6	13	1	30 (2)	23.3%	0
Glaxo SmithKline	0	11	0	3	12	1	27 (3)	40.7%	3
Boehringer Ingelheim	0	4	0	6	10	7	27 (3)	14.8%	2
Pfizer	0	5	2	4	12	2	25 (4)	20.0%	0
Johnson & Johnson	0	8	0	1	11	3	23 (5)	34.8%	5
Sanofi	1	6	1	4	8	2	22 (6)	31.8%	3
Incyte	0	7	1	1	8	4	21 (7)	33.3%	0
Bristol Myers Squibb	0	6	4	3	7	1	21 (7)	28.6%	3
Novartis	0	3	3	1	13	0	20 (8)	14.3%	2
AbbVie	0	3	0	2	9	4	18 (9)	16.7%	1
Amgen	0	4	2	2	6	3	17 (10)	23.5%	0

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 2025

Successful positive pivotal trials were localized to 40 individual positive pivotal studies and covered 24 diseases within the A/I portfolio. The total number of trials deviates from the stable number of 42 trials recovered from the past three years (2023, 2022, and 2021). The aggregation of the successful trials by disease discloses 57 positive pivotal results, composed of 38 registration and 19 expanded indication studies (Figure 4). After sliding to second place in last year's valuation, psoriasis has returned to first place and was forthcoming with one expanded indication and eight registration studies.

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Also demonstrating sizable changes in yearly quantities, other inflammatory arthritis (psoriatic arthritis and spondyloarthropathies) generated fourfold improvement as it rated second with eight pivotal trials (three expanded indication and five registration). Under these two high-performing pivotal disease areas, matching values was found for atopic dermatitis (four) and food allergy (four) this year. Each of the results was equally partitioned by two registration and two expanded indication trials but displayed different forms of movements from the prior year's results. Differences from last year outlined that atopic dermatitis had dropped in notoriety as it reported half of the total completed pivotal trial results from 2023 (eight; two registration and six expanded indication). Also expressing fewer positive pivotal results were cystic fibrosis, anti-aging, and urticaria.

Separate from these decreases, food allergy made an appearance as it disclosed positive results for the first time in such an analysis. Other indications that did not contain results last year but were added to this year's evaluation were primary biliary cholangitis (three; two registration and one expanded indication), pulmonary fibrosis (three registration), pemphigoid (two; one registration and one expanded indication), eosinophilic esophagitis (one registration), lupus (one expanded indication), nasal polyps (one expanded indication), non-cystic fibrosis bronchiectasis (one registration), and polymyalgia rheumatica (one expanded indication).

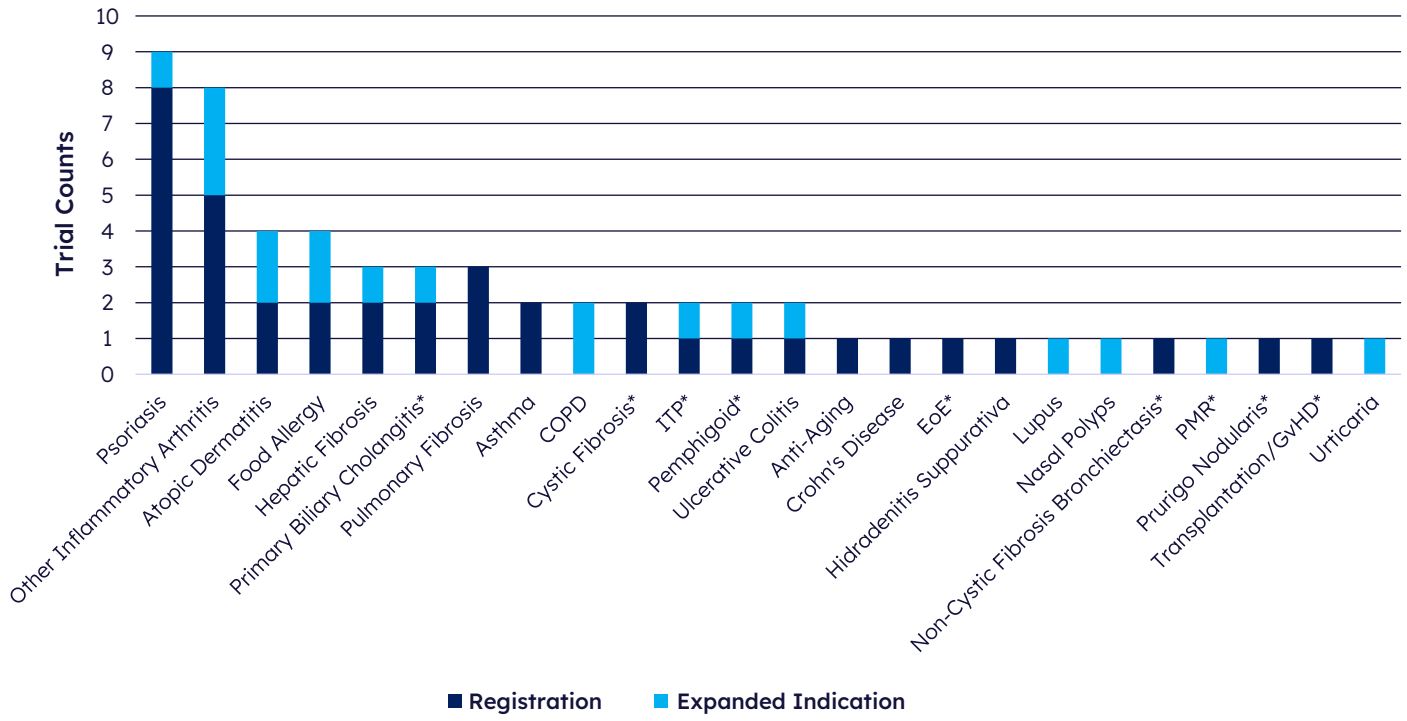
Beyond new additions, several diseases also saw rearrangement in the order of total pivotal trials. Outside the top two diseases that expressed significant movement, both hepatic fibrosis (three; two registration and one expanded indication) and immune thrombocytopenia (two; one registration and one expanded indication) increased by one due to each completing an expanded indication trial.

Additionally, seven indications represented no change in annual total pivotal amounts and were inclusive of asthma (two), COPD (two), ulcerative colitis (two), Crohn's disease (one), hidradenitis suppurativa (one), prurigo nodularis (one), and transplantation/GvHD (one). Note, graft-versus-host disease (GvHD) is considered a rare indication and presently is categorized within the transplantation disease coverage. The corresponding, positive registration trial was a Phase III study evaluating treatment for acute GvHD with gastrointestinal involvement.



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



*Rare disease

Source: Trialtrove, February 2025

The A/I drug analysis identifies those which are currently in pipeline status and were noted by positive pivotal trials. Designation for MOA novelty was further assigned to the drugs represented based upon whether they were unique (Table 10). The pipeline status drugs included a conglomeration of top 20 pharma (four) along with AOP sponsors (seven) and summed a total of 11 drugs. Three of the listed therapies included novel MOAs and were included in multinational trials. The remaining eight drugs had non-novel MOAs and therefore are considered as alternative therapies for drugs that have reached the market. Two of these "me-too" drugs (picankibart and solvepenib) were utilized in trials that were solely run in China by sponsors Innovent Biologics and Hutchmed, in that order.

A review of the pivotal trial diseases highlights that the referenced therapies are predominantly directed at dermatological indications. More than this, dermatology overshadowed other A/I disease areas as 18 pivotal trials were completed and spanned six individual diseases. The total amount of these trials is exactly double that observed in both 2022 and 2023 (nine).

An additional rise in respiratory drug trials was noted as these volumes showed completion of 11 trials treating subjects with pulmonary fibrosis, asthma, COPD, and others. The MOAs linked with these drugs included cytokine antagonists, a cytokine signaling antagonist, a protein kinase inhibitor, a microbiome modulator, and a NOX inhibitor. All the novel drugs also demonstrated unique MOAs for a cysteine protease inhibitor, cytokine antagonist, and PDE inhibitor.

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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
brensocatib	Insmed	Non-Cystic Fibrosis Bronchiectasis*	Cathepsin C inhibitor	Yes
cendakimab	Bristol Myers Squibb/ Celgene	EoE*	IL-13 antagonist	Yes
nerandomilast	Boehringer Ingelheim	Pulmonary Fibrosis	PDE-4 inhibitor; PDE inhibitor	Yes
depemokimab	GlaxoSmithKline	Asthma	IL-5 antagonist	No
deupirfenidone, PureTech Health	PureTech Health	Pulmonary Fibrosis	TNF alpha antagonist; TNF ligand antagonist	No
icotrokinra	Johnson & Johnson/ Janssen R&D	Other Inflammatory Arthritis; Psoriasis	IL-23 antagonist; IL-23 R antagonist	No
izokibep	Acelyrin	Hidradenitis Suppurativa	IL-17A antagonist; T helper cell inhibitor	No
MaaT-013	MaaT Pharma	Transplantation/GVHD*	Microbiome modulator, faecal	No
picankibart	Innovent Biologics	Psoriasis	IL-23 antagonist	No
setanaxib	Calliditas Therapeutics/ Genkyotex	Hepatic Fibrosis; Primary Biliary Cholangitis*	NADPH oxidase 1 inhibitor; NADPH oxidase 4 inhibitor; IO therapy	No
solvepenib	Hutchmed	ITP*	Syk TKI	No

*Rare Disease
Gray fill: China-only trial

Source: Trialtrove and Pharmaprojects, February 2025

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

Measuring the third-highest clinical trial activity during this period, CNS trials had the most diverse number of sponsors out of the three top TAs (18). The top sponsor analysis assessed the success rate, positive pivotal trial counts, and total trial tallies across study Phases I–III/IV while ranking sponsors accordingly (Table 11). Eleven of these 18 sponsors are classed as top 20 pharma, where the alternate seven were denoted as AOP. Moreover, the aggregated count of these trials totaled 264 trials and yielded a 55% increase over the prior year's value (170). Despite an increase in CNS trial completions, overall trial success did not experience the same upsurge. Year over year, the positive trial results differed by 12 where 34 were found for 2023 and 46 in 2024. Although the total trial quantities increased and the positive trial counts saw slight growth, the success metric saw a decline for CNS trial outcomes (20.1% in 2023 vs. 17.4% in 2024).

A breakdown of top CNS sponsors illuminated a disparity in the first (BI; 29) and last place (Eisai; eight) total trials. Deepening the evaluation, varying rates of success were found across each of the sponsors, where BMS possessed the greatest positive results (55.6%), followed by Otsuka (44.4%), Lundbeck (36.4%), and Vertex (36.4%).

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BMS trial results were fortified in the top CNS sponsor analysis following its acquisition of Karuna Therapeutics, a company specializing in neuroscience therapies.[4] Alongside BMS, five other sponsors were introduced in the report and consisted of Merck & Co., Pfizer, Novartis, Qilu Pharma, and Eisai. Many return sponsors had a heavy presence in the results as change was found among the trial and success ratings. The most notable, progressive adjustment in ranking was found for BI (first vs. sixth), AbbVie (second vs. seventh), UCB (fourth vs. seventh), and Biogen (fifth vs. eighth). In contrast, a drop in trial ranking was determined for J&J (fifth vs. third), Eli Lilly (sixth vs. second), Otsuka (ninth vs. fourth), and AZ (eighth vs. first). Transitioning to the positive pivotal column data, only six top sponsors interpreted positive results in their pivotal trials and combined to sum 11 of the entire 46 positive trials. These details reveal that two-thirds of the top sponsors did not publicly supply positive outcomes for their pivotal trials.



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Table 11. Top sponsors with completed central nervous system (CNS) trials, by success rates and positive pivotal trial counts

Sponsor	Early positive outcome	Positive outcome/ primary endpoint(s) met	Outcome indeter-minate	Negative outcome/ primary endpoint(s) not met	Outcome unknown	N/A	Total trials (rank)	Success rate	Positive pivotal trials (count)
Boehringer Ingelheim	0	6	4	7	2	10	29 (1)	20.7%	0
AbbVie	0	5	3	1	12	7	28 (2)	17.9%	3
Roche	0	2	4	7	5	2	20 (3)	10.0%	1
Merck & Co.	0	0	0	5	12	3	20 (3)	0.0%	0
UCB	0	0	2	3	6	6	17 (4)	0.0%	0
Biogen	0	3	1	2	6	4	16 (5)	18.8%	0
Johnson & Johnson	0	4	1	4	5	2	16 (5)	25.0%	1
Eli Lilly	0	2	4	6	2	0	14 (6)	14.3%	0
Novartis	0	3	1	2	6	2	14 (6)	21.4%	1
Takeda	0	2	3	4	4	1	14 (6)	14.3%	0
Lundbeck	0	4	2	1	4	0	11 (7)	36.4%	0
Vertex	0	4	0	0	4	3	11 (7)	36.4%	3
AstraZeneca	0	0	1	5	3	1	10 (8)	0.0%	0
Bristol Myers Squibb	0	5	0	1	2	1	9 (9)	55.6%	2
Otsuka Holdings	0	4	1	4	0	0	9 (9)	44.4%	0
Pfizer	0	0	1	3	2	3	9 (9)	0.0%	0
Qilu Pharma-ceutical Co.	0	0	0	0	0	9	9 (9)	0.0%	0
Eisai	0	2	0	0	4	2	8 (10)	25.0%	0

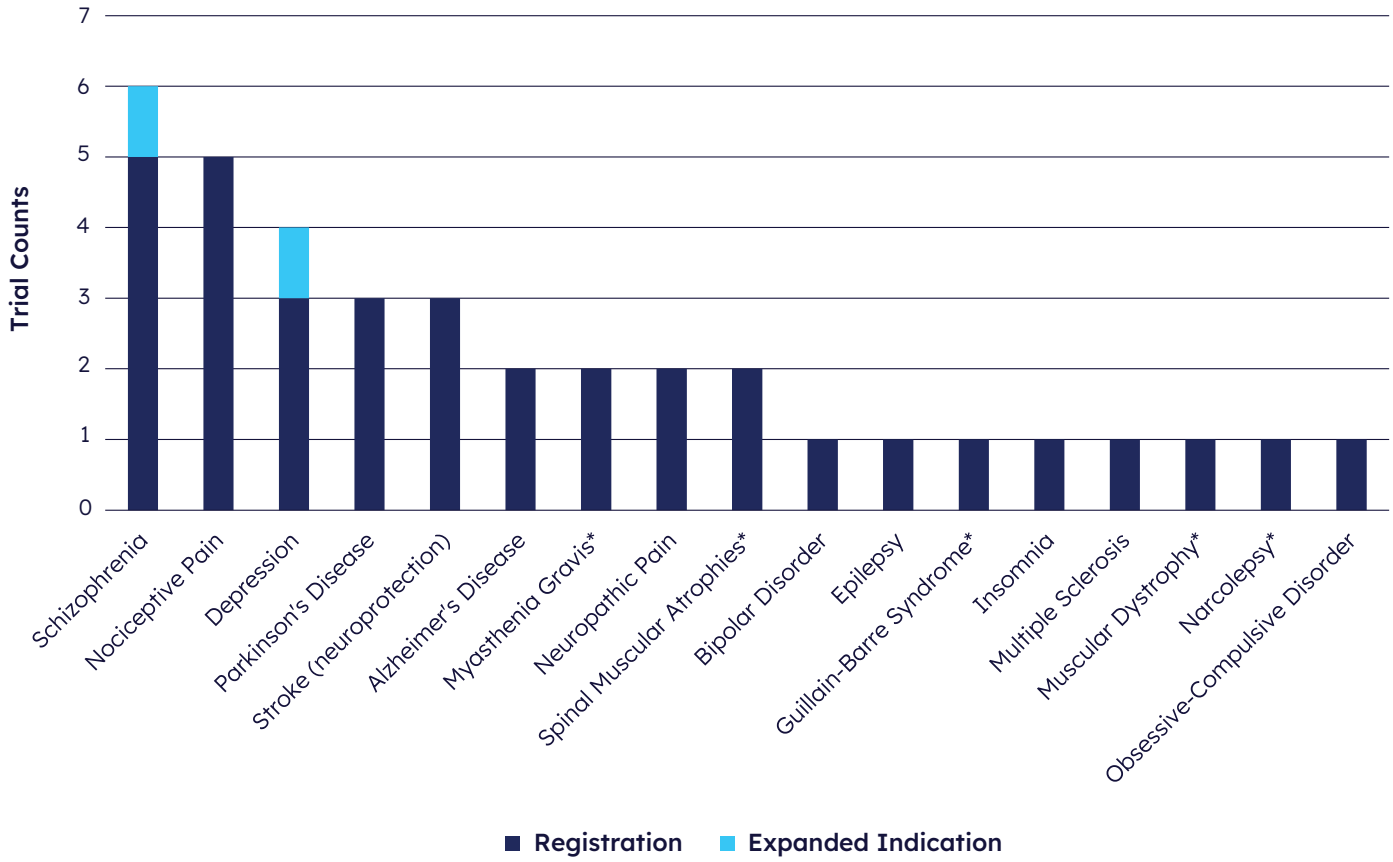
Note: Indeterminate designation is assigned to trials when the outcome is neither clearly positive nor negative. Unknown is assigned to trials that have yet to report full results for their primary endpoints. NA indicates trials with no efficacy/safety outcomes to evaluate.

Source: Trialtrove, February 2025

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Repositioning to examine all positive pivotal trial data by disease, filing type, and regardless of top sponsorship, 17 diseases were captured in Figure 5. The annual disease counts remain indifferent from the previous results that also emphasized 17 diseases; however, five of the 17 indications are disclosed as rare. Each of the other 12 diseases are more prevalent indications with high disease burdens and are also seeking to bring new therapies to market, as the main population were registration studies. Positive pivotal schizophrenia trials have shown a rise in this report as they took the lead, reflecting five registration trials and one expanded indication study. Nociceptive pain continues to maintain presence in second place as it grew from four to five registration trials during the yearly timeframe. The pivotal results also showed that 80% of the nociceptive pain trials were evaluating non-opioid therapeutics for the treatment of acute or postoperative pain management. These counts are consistent with the findings found in Table 3, which noted that nociceptive pain had heightened numbers of late-phase clinical trials. On the contrary, Alzheimer's disease alluded to many of the trials being early phase (I–II), non-pivotal trials and were supported by obtaining surrogate efficacy endpoints. A continual pattern of registration trials dominating the CNS landscape is apparent as 35 trials were listed with the appropriate filing type.

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



*Rare disease

Source: Trialtrove, February 2025

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MOA novelty, undisclosed MOA or China-only trials

An investigation of positive pivotal trials revealed many pipeline status drugs for such CNS indications and outlined each as to whether they contain novel or non-novel MOAs (Table 12). The results are reflective of 15 drugs across 11 individual diseases as effective new and alternative therapies. Drugs with previously proven MOAs composed the greatest proportion of these therapies, many of which included partial dopamine agonists, calcium channel inhibitors, voltage-gated sodium channel inhibitors, serotonin (5-HT) modulators, a human tissue plasminogen kinase derivative, and others with undisclosed mechanisms. Therapeutics with unspecified MOAs are indicated to treat bipolar disorder and stroke (neuroprotection), respectively.

In addition, two pharmaceuticals were included in China-only trials, with one sponsored by Angde Biotech and the second by Lumosa Therapeutics (originator) with Shanghai Pharma (licensee). Both schizophrenia and stroke (neuroprotection) returned a total of three drugs in development; however, two of the drugs (brilaroxazine and evenamide) contained MOA novelty for schizophrenia.
MOA novelty was assigned to brilaroxazine for its dopamine D4 receptor agonist properties and evenamide being a sodium channel antagonist and voltage-gated sodium channel antagonist. Each of these drugs was joined with novel products for rare Guillain-Barre syndrome (ANX-005, a humanized IgG4 MAb) as well as Parkinson's disease (tavapadon, a partial dopamine agonist).

Different from years past, CNS pipeline drugs did not include an abundance of reformulated drugs. Only four out of 15 products emphasized such modification to the composition of select therapies, namely fixed-dose combinations for two (D-cycloserine+lurasidone and naproxen+pregabalin), oral reformulation of N-methyl amisulpride, and an optimized nanoparticle reformulation of nimodipine. Each of the related pivotal trials was run in the United States only and saw positive results.

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
ANX-005	Annexon Biosciences	Guillain-Barre Syndrome*	C1Q inhibitor; Complement convertase inhibitor; Complement factor inhibitor	Yes
brilaroxazine	Reviva Pharmaceuticals	Schizophrenia	5-HT1A R agonist; 5-HT2A R antagonist; 5-HT2B R antagonist; 5-HT2C R antagonist; 5-HT6 antagonist; 5-HT7 R antagonist; Dopamine D2 R agonist; Dopamine D3 R agonist; Dopamine D4 R agonist	Yes

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Drug name	Sponsor	Disease	Mechanism of action	Novel
evenamide	Newron	Schizophrenia	Sodium channel antagonist; Voltage-gated sodium channel antagonist	Yes
tavapadon	AbbVie	Parkinson's Disease	Dopamine D1 R agonist; Dopamine D5 R agonist	Yes
D-cycloserine + lurasidone, NeuroRx	NRx Pharmaceuticals {NeuroRx}	Bipolar Disorder	Unidentified pharmacological activity	No
tolebrutinib	Sanofi	Multiple Sclerosis*	BTK inhibitor	No
trigriluzole	Biohaven	Obsessive-Compulsive Disorder	Dopamine R agonist; Glutamate antagonist; Voltage-gated sodium channel antagonist	No
apitegromab, Scholar Rock	Scholar Rock	Spinal Muscular Atrophies*	Growth and differentiation factor 8 antagonist	No
nipocalimab	Momenta Pharmaceuticals (now Johnson & Johnson)	Myasthenia Gravis*	Fc fragment of IgG R and transporter antagonist	No
cyclobenzaprine, TONIX	TONIX Pharmaceuticals	Neuropathic Pain	5-HT2A R antagonist	No
reteplase, Angde Biotech Pharmaceutical	Angde Biotech Pharmaceutical	Stroke (neuroprotection)	Tissue-type plasminogen activator stimulant	No
LT-3001	Lumosa Therapeutics/ Shanghai Pharmaceuticals Holding Co.	Stroke (neuroprotection)	Unidentified pharmacological activity	No
nimodipine, Grace Therapeutics	Acasti Pharma/Grace Therapeutics	Stroke (neuroprotection)	Calcium channel antagonist; L-type calcium channel antagonist	No
naproxen + pregabalin, Xgene Pharmaceuticals (tablet)	Xgene Pharmaceutical	Nociceptive Pain	Calcium channel antagonist; Cyclooxygenase inhibitor	No
N-methyl amisulpride, LB Pharmaceuticals	LB Pharmaceuticals	Schizophrenia	Dopamine D2 R antagonist, D3 R antagonist	No

*Rare disease
Gray fill: China-only trial
HT= hydroxytryptamine; BTK=bruton tyrosine kinase; R=receptor

Source: Trialtrove and Pharmaprojects, February 2025

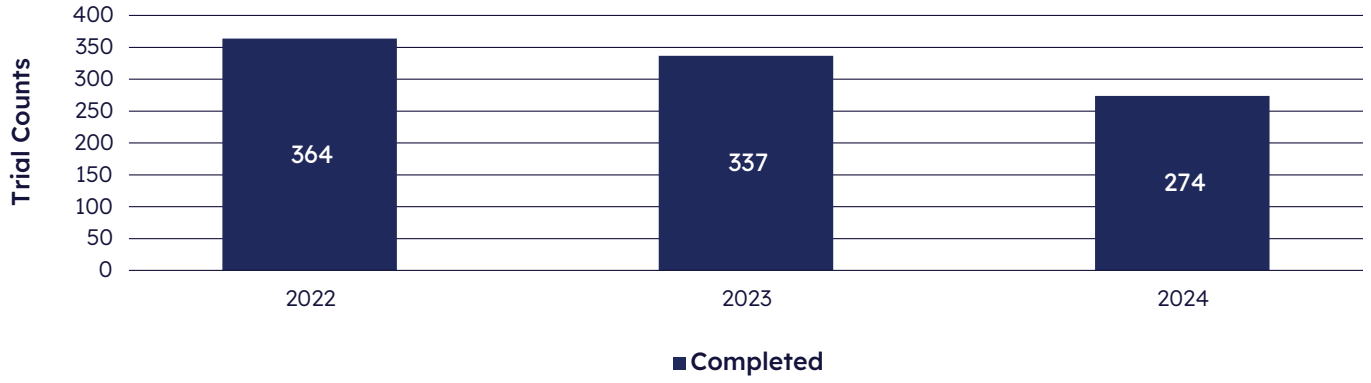
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Current Events in Clinical Trials and Related Impacts

Amid a multiplicity of geopolitical disruptions, natural disasters, and national and international elections throughout 2024, the industry has carefully navigated several obstacles in clinical trial operations. Many of these events took on the forms of devastating wildfires, hurricanes, or earthquakes, while others were caused by volatility in select countries and impeded the process of companies moving lifesaving therapies to market.

A separate analysis was warranted to expound upon annualized trial volumes and related outcomes arising from those impacted by the conflict in Eastern Europe (Figure 6). Several effects were found through a granular evaluation of trials conducted in the last three years and involved the countries of Russia and Ukraine. These disruptions pressured sponsors to pivot and find new locations to conduct studies, maintain available sites, halt recruitment in effected areas, and retain subjects due to large numbers of discontinuations. In addition to this, obtaining adequate study resources was also a point of struggle as supply chain issues arose resulting from the displacement of critical study components, global inflation, and material shortages. Over the last three years, a decline in the number of completed trials (274 vs. 364) was observed for those inclusive of the referenced countries. Much can be attributed to many sponsors, both top 20 and AOP, communicating their intentions to not initiate new trials in the affected countries nor to enroll new subjects from such countries into ongoing studies. As a result, the yearly trial totals where these countries were selected have diminished. Coupled with this drop, multiple completed studies expressing related difficulties were revealed for 14 trials completing in 2022, 12 completing in 2023, and 26 completing in 2024 (data not shown). The data represented suggest a continual lowering of trials taking place in these Eastern European countries as the war in Ukraine persists. However, as hope for an armistice draws closer and optimism for peaceable negotiations prevails, a change in trial landscape could be imminent for these two countries.

Figure 6. Trial counts for studies including the countries of Russia and/or Ukraine 2022–2024



Source: Trialtrove, February 2025

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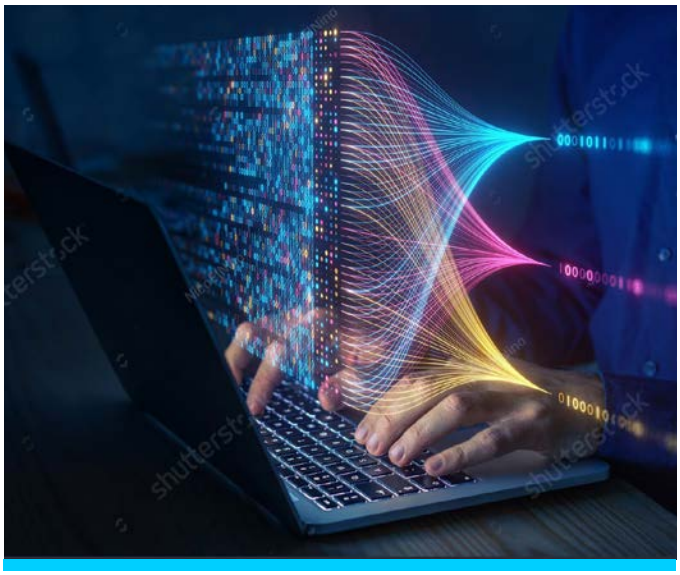
Key Takeaways

Our annual completed trial analysis emphasized moderate, broad-based growth throughout the course of 2024. The stable percentages of progression over consecutive periods demonstrated modest advancement for all sponsors as new heights were reached for trials completed (4,903) since the beginning of our Annual Completed Trials reports. These augmented results were influenced by many prominent and mid-tier TAs, which are in large part comprised of chronic indications, as found in Tables 2 and 3. Moreover, the top 20 pharma sponsor rankings showed meaningful rearrangement as Merck & Co. moved into the top slot and had key involvement in two of the three top TAs (second in oncology and third in CNS). Also listed in the top five sponsors, Pfizer and AZ were placed in rankings for each of the top three TAs.

Conflicting with the past year's analyses and despite the robust volume of completed trials, new pipeline drugs found for the top three TAs did not undergo the same increase but rather saw fewer or matched numbers of drugs in development and lower success rates for completed studies (34.2%, oncology; 25.8%, A/I; 17.4%, CNS). Although the amount of pipeline drugs did not rise above the previous term's results, CNS pivotal drugs showed improvement with the introduction of four drugs with novel MOAs. Further, AOP sponsors established presence as they pervaded the list of companies disclosing positive pivotal results for top TA pipeline drugs (38 of 45 companies; 39 drugs). These counts were hardly conflated with top 20 sponsors and only disclosed seven of 45 companies and eight associated drugs in pipeline. The AOP sponsors proved a crucial role in the drug development process in reflection of these results.

As focus is drawn away from pandemic-related disturbances and underlying geopolitical disruptions abound, the industry adapts to maintain homeostasis. The pinnacle volumes of trials surged throughout 2024, but trial success vacillations, terminations, and decreased trial completions for affected Eastern European countries were notable amid international conflicts. Several logistical factors, due to the war in Ukraine and surrounding countries, contributed to terminations and fewer completed trials for affected areas. These impacts have proven formidable obstacles for clinical trials and sponsors alike, and such barriers could lead companies to expand trial country selection until the conflict subsides.

The past year marked many firsts for industry-sponsored trials and continually exhibits a proclivity for evolution as new heights are reached. These measures reflect a strong pulse in the clinical trial industry but also highlight new areas for future change.



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Drew currently supports clinical trial intelligence for the cardiometabolic, vaccine, infectious disease, and genitourinary therapy areas at Citeline. He excels in conducting in-depth data analyses for a variety of applications and utilizes these methods in constructing detailed Ask the Analyst responses. Additionally, through his membership on our analyst bench team, Drew has assisted on a variety of bespoke consulting projects to serve clients. Prior to joining Citeline in 2021, he also conducted research in immunology, lupus, and cell apoptosis. The study focused on determining relative gene expression in cell culture derived from animals that had contracted lupus.



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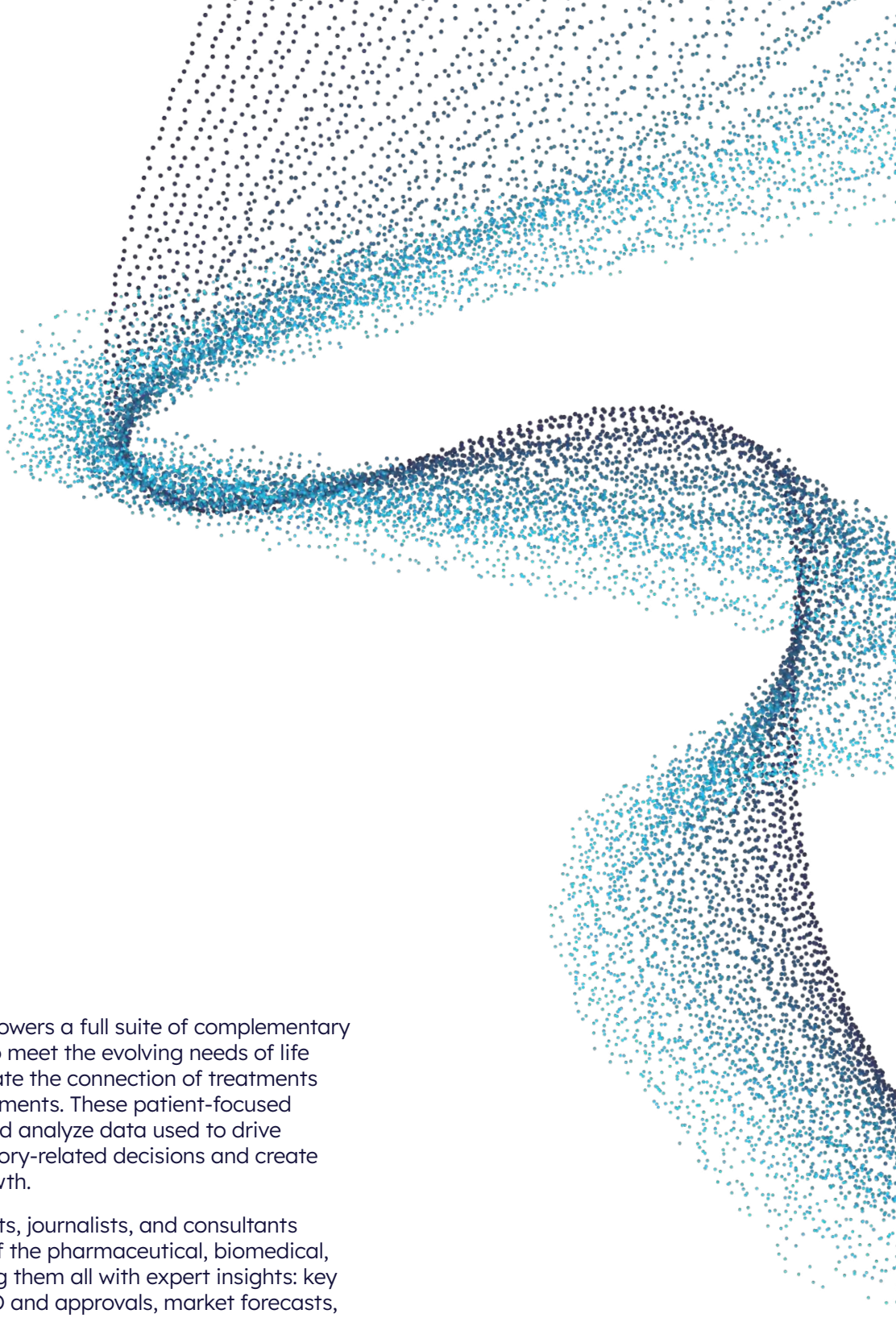
Appendix

Mechanisms of Action and Disease Terms Abbreviations

5-HT	5-hydroxytryptamine
ALL	acute lymphocytic leukemia
AML	acute myelogenous leukemia
BTK	Bruton tyrosine kinase
CDK	cyclin-dependent kinase
CLL	chronic lymphocytic leukemia
CML	chronic myelogenous leukemia
COPD	chronic obstructive pulmonary disease
CSF-1	colony-stimulating factor 1
EoE	eosinophilic esophagitis
HDAC	histone deacetylase
IL	interleukin
IO	immuno-oncology
ITP	immune thrombocytopenia
NHL	non-Hodgkin's lymphoma
NSCLC	non-small cell lung cancer
PDE	phosphodiesterase
PMR	polymyalgia rheumatica
R	receptor
SCLC	small cell lung cancer
TK	thymidine kinase
TKI	tyrosine kinase inhibitor
TNF	tumor necrosis factor
VEGF	vascular endothelial growth factor

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