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TOP FIVE STUDIED DISEASES IN 2025

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Executive Summary

Phesi's annual Top Five Studied Diseases report tracks the world's most studied diseases using Phesi's award-winning, AI-powered Trial Accelerator™ which leverages contextualized real-world data from more than 300 million patients. Now in its fifth year, the report is based on 65,892 recruiting clinical trials from across the world and in addition to highlighting the most studied diseases, it examines changes in Phase II trial attrition rates and monitors the top five countries hosting clinical trial sites.

Key findings:

- Breast cancer is the most studied disease area for the fifth year running, followed by solid tumors, stroke, prostate cancer and non-small cell lung cancer
- Obesity ranks in sixth position, as a rising challenger to the five most studied diseases
- Trial attrition rates finally show signs of post-Covid recovery but remain high with one in four trials terminated in Phase II
- The United States leads global clinical trial activity across the top five indications
- China records the fastest growth in the number of recruiting clinical trial sites

Key takeaways:

- Sustained clinical research in oncology and in particular in breast cancer is encouraging but the complex nature of disease means many unmet needs still need to be addressed
- As the industry moves beyond Covid, sponsors must watch out for the next disruptor of the clinical landscape. GLP-1s, for example, could shift the focus to the prevention and treatment of inter-related diseases
- Using AI and the growing body of contextualized real-world data to power clinical data analytics, sponsors can improve decision making from feasibility and design to trial execution, reducing the cost, patient and investigator burden and complexity of trials

Top Five Studied Disease Areas

Phesi's annual report into the world's most studied diseases shows that breast cancer across all subtypes – from triple-negative to PIK3CA – remains the world's most studied disease for the fifth year running. The top five also includes solid tumors, stroke, prostate cancer and non-small cell lung cancer (NSCLC) (table 1) once again this year.

Table 1 – Clinical development: Top five most studied diseases

2021 (75,020 trials)	2022 (80,917 trials)	2023 (65,749 trials)	2024 (67,469 trials)	2025 (65,892 trials)
Breast cancer	Breast cancer	Breast cancer	Breast cancer	Breast cancer
COVID-19	COVID-19	Solid tumors	Solid tumors	Solid tumors
Non-small cell lung cancer	Prostate cancer	Stroke	Stroke	Stroke
Solid tumors	Solid tumors	COVID-19	Prostate cancer	Prostate cancer
Multiple myeloma	Stroke	Prostate cancer	Non-small cell lung cancer	Non-small cell lung cancer

All data from Phesi Trial Accelerator ™

Obesity remains just outside the top five most studied diseases in 2025, ranking sixth. A separate Phesi analysis in September 2025 showed that more than 100 diseases are being investigated in connection with GLP-1 use, reflecting growing interest in obesity as a comorbidity in a wide range of diseases. This expansion indicates that obesity is the disease area most likely to enter the top five within the next one to two years as rising GLP-1 usage will also influence clinical development in other fields, as obesity is an important comorbidity and weight loss may alter study parameters, dosing and endpoints.

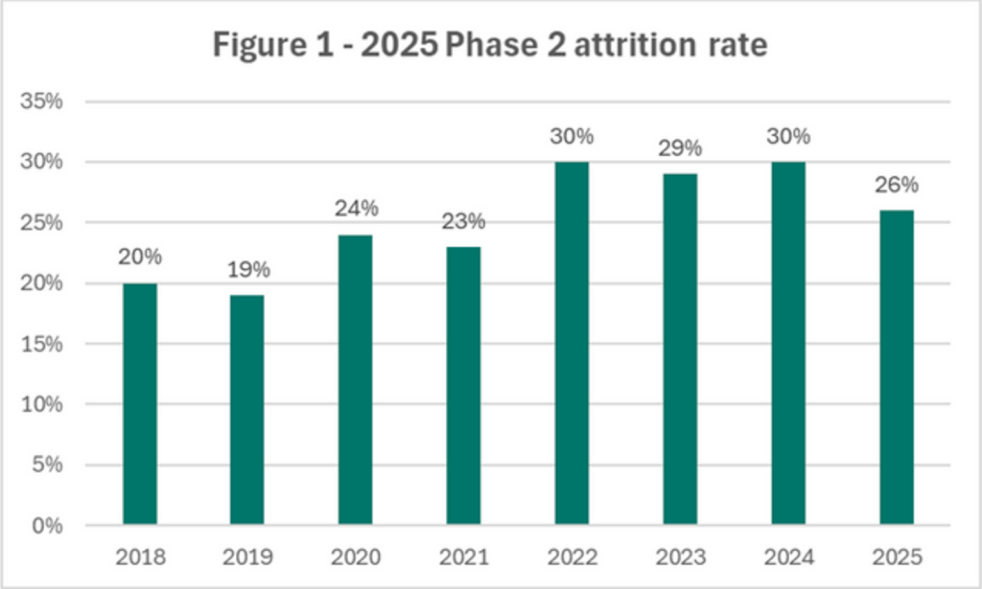
Breast cancer, the top killer among women with cancer, continues to dominate because researchers have a far deeper understanding of biomarkers. There are still many unmet needs to be addressed, such as in triple negative breast cancer, but sustained research investment in this devastating disease is encouraging.

Although the pandemic is still having lingering effects as a disruptor, sponsors should now be looking ahead to what the next disruptor might be. The broader adoption of GLP-1s, for example, could reshape the clinical development landscape as the focus shifts towards prevention and treating clusters of related disease.

Trial Attrition Rates

Phesi's annual global analysis is one of the few studies dedicated to tracking Phase II attrition rates, because of the pivotal role in clinical development. This important metric can highlight a slowing down of the rate at which new therapies reach market and the rising development costs for industry.

This year's analysis shows an encouraging fall in trial attrition rates to a four-year low, although still higher than pre-pandemic levels. With a quarter (26%) of trials terminated in Phase II, this remains an unacceptably high figure and will inevitably have a knock-on effect on Phase III trials and a significant negative impact on ROI across the biopharmaceutical industry. By comparison, in 2024, 31% of Phase II trials were terminated, up from 29% in 2023 and around 20% before Covid-19 (fig.1). It is important to note that the rising Phase II attrition rate during COVID was not caused by failing COVID related development programs.

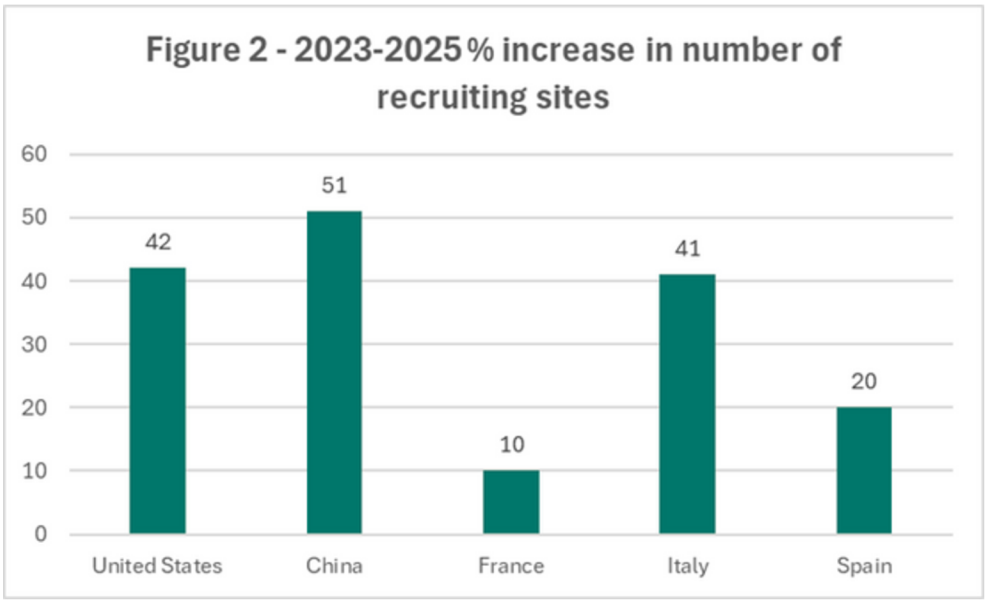


Top Five Countries

Phesi's country-level analysis shows the United States continues to host the largest number of recruiting clinical trial investigator sites overall and for each of the five most studied diseases. China ranks second for all of the top five except prostate cancer, where Canada takes second place. China also recorded the strongest growth in investigator site numbers between 2023 and 2025, increasing by 51% compared with 42% for the US (fig.2). France, Italy and Spain complete the top five countries for recruiting investigator sites.

Top Five Countries cont...

Sponsors remain under pressure from macroeconomic constraints, pricing challenges and increased activity in regions such as China. The data also shows the effect of renewed emphasis from regulators, including the FDA, on country-specific representation. This can lead to well-known and high-profile investigator sites quickly becoming saturated as sponsors seek them out. However, by leveraging AI and real-world data to provide dynamic insights into country and investigator site selection, it is possible to identify investigator sites elsewhere that may have a shorter clinical trial and enrollment history, but have capacity and have delivered high quality data in previous trials.



Overcoming the Challenges

Increasing competition for investigator sites and patients, regulatory changes and acceptance of digital patient data mean it is vital for sponsors to be led by insight powered by big data and AI, rather than instinct. A considerable volume of contextualized, real-world data exists today to power clinical data analytics and ensure every aspect of patient profiling, trial design, including the most suitable countries and investigator sites, is optimized and proven to reduce the cost, patient burden and complexity of trials.

Phesi leverages its award-winning Trial Accelerator™ platform with the world's largest contextualized clinical trial database to deliver clinical data science led feasibility solutions that provide a deeper understanding of the patient, their disease pathway and who is treating them, ensuring greater precision, insight and certainty in clinical development.

At the heart of every solution is the Digital Patient Profile. This is a picture of the key characteristics specific to the indication and target patient population including age, sex, comorbidities, outcome measures and concomitant medications. It is used to optimize program and protocol design, enhance the probability of technical and regulatory success (PTRS) and eliminate unnecessary and costly protocol amendments. In combination with Phesi's Patient Access Score - a unique performance measure – Phesi guides sponsors in selecting the best performing investigator sites, resulting in shorter cycle times and reduced patient burden.

A Digital Patient Profile is the basis of Digital Twin analysis - a representation of typical patients in a specific population generated from a vast body of real-world patient data from identical or similar trials, allowing researchers to simulate, model and predict safety and efficacy outcomes as a historic control arm. Other use cases include External Control Arms to reduce the number of patients undergoing testing in trials. Phesi's disruptive innovation has helped many world-leading biopharmaceutical companies to deliver lifesaving medicines to patients.

Example of a Digital Patient Profile for Breast Cancer with PIK3CA

Phesi Digital Patient Profile: Breast cancer with PIK3CA		Digital Baseline Patient Characteristics	Data from Real World Sources	
			# pts	#cohorts
Demographic	Age (yrs), median (min - max)	60 (40 - 79)	17,270	257
Demographic	<50 yrs	39%	14,046	45
Demographic	>50 yrs	60%	10,315	30
Race	White	76%	22,515	102
Race	Black	5%	13,549	85
Race	Asian	20%	16,633	95
Ethnicity	Hispanic	10%	4,645	32
Metastatic site	Liver	43%	9,450	84
Metastatic site	Bone	41%	10,637	79
Metastatic site	Lung	31%	4,824	60
Metastatic site	Bone only	19%	5,968	45
Performance status	ECOG 0	59%	17,164	158
Performance status	ECOG 1	33%	17,071	155
Performance status	ECOG 2	2%	11,062	67
Performance status	ECOG 3	0%	1,967	2
Histology	Invasive ductal	75%	12,493	58
Histology	Invasive lobular	10%	11,101	38
Histology	Grade 1	10%	20,688	84
Histology	Grade 2	45%	20,688	84
Histology	Grade 3	44%	26,264	99
Prior treatment	Tamoxifen	43%	2,892	44
Prior treatment	Aromatase inhibitor	48%	9,403	49
Prior treatment	Trastuzumab	19%	10,221	41
Prior treatment	Fulvestrant	23%	2,215	35
Prior treatment	Taxane	34%	1,228	32
N stage	N0	46%	11,428	62
N stage	N1	37%	4,926	44
N stage	N2	9%	4,926	44
N stage	N3	6%	7,625	42
T stage	T1	30%	4,201	30
T stage	T2	59%	5,569	46
T stage	T3	10%	5,512	44
T stage	T4	4%	3,454	29
Biomarker	ER+ and/or PgR+	69%	4,303	34
Biomarker	HER2-	69%	28,402	92
Biomarker	HER2+	23%	5,512	44
Biomarker	ER- PgR- HER2- (triple negative)	12%	25,754	76
Biomarker	PIK3CA mutated	27%	6,170	66
Biomarker	PIK3CA wild type	53%	3,820	41

Phesi's Digital Patient Profile catalog today holds 44 patient profiles across disease areas including oncology, respiratory, metabolic, dermatology, inflammation, CVS and CNS, and all of the top five most studied diseases, including:

- Breast cancer: PIK3CA, HER2-Positive
- NSCLC: general NSCLC and KRAS mutated G12C and G12D NSCLC, and NSCLC-EGFR
- Stroke: general and Acute Ischemic Stroke
- Prostate cancer: metastatic, Castration Resistant
- Pancreatic cancer: KRAS mutated, G12D
- Parkinson's Disease: Deep Brain Stimulation
- Obesity-related indications: MASH, Alzheimer's, Acute Coronary Syndrome (ACS), Osteoarthritis, GLP-1

For further information or to access Phesi's Digital Patient Profile Catalog and recent case studies please get in touch via email: info@phesi.com

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