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Industry pulse: Life sciences

A snapshot of the most impactful trends—from therapeutic developments to evolving care models—that are influencing the future of life sciences.

A Definitive Healthcare report

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What's next for medical progress in the U.S.?

The life sciences industry plays a pivotal role in our economic growth and health, driven by innovation that not only fuels progress but also transforms lives. As we enter what many call a golden age of medicine, it's important to understand the forces shaping its future, from the emergence of new treatments to the evolving care settings that influence how technologies reach patients.

These developments are unfolding against the backdrop of a changing political landscape, where new regulatory priorities and policy shifts under the Trump administration add a layer of uncertainty to an already complex environment, posing both familiar and emerging challenges for stakeholders.

In this report, we highlight key developments to watch: The rise of artificial intelligence in clinical trials, the shift of surgical procedures to ambulatory surgical centers (ASCs), and significant advances in gene editing. We also examine how changes at key agencies like the FDA could affect how innovations reach the market.

These areas not only showcase the ongoing innovation in the pharmaceutical and medical device sectors but also carry important implications for patient care, regulatory frameworks, and market dynamics, creating fertile ground for opportunity and disruption. Our goal is to provide a clear snapshot of these developments, offering insights to help organizations understand the current landscape and stay attuned to the shifts occurring in the industry.

KEY INSIGHTS OVERVIEW

- **Insight 1:** The rise of artificial intelligence in clinical trials is poised to transform therapeutic development, speeding up the path from research to real-world application.
- **Insight 2:** The landscape of sites of service for surgical procedures is changing dramatically, with more procedures shifting to ASCs.
- **Insight 3:** Advances in gene editing are set to deliver a wave of new treatments for complex diseases, marking a significant leap toward personalized medicine.
- **Insight 4:** The transition to a new administration introduces policy shifts that could impact regulatory processes and development timelines, emphasizing the need for proactive planning.

Insight 1: The rise of AI in clinical trials is poised to transform therapeutic development

Drug trials face a high failure rate, with only 13.8% of drug development programs ultimately leading to approval.[1] For those that do succeed, the average time from the start of clinical testing to marketing is 7.5 years.[2] Moreover, the costs associated with trials supporting FDA approval can range from $19 million to as much as $2.9 billion, placing a significant financial risk on pharmaceutical sponsors.[3-4] The growing complexity of trial designs further exacerbates these high costs and long timelines.

INCREASING COMPLEXITY OF TRIAL DESIGNS OVER THE PAST TWO DECADES

Category	2001–05	2011–15	2016–20	20-year overall rise
Endpoints*	7	13	22	214%
Procedures*	110	187	263	139%
Eligibility criteria*	31	30	30	–3%
Countries*	6	9	15	150%
Data points collected*	494,236	929,203	3,453,133	599%

Fig. 1 Source: Tufts Center for the Study of Drug Development. *Mean of total numbers

In addition, federal agencies like the National Institutes of Health (NIH) fund much of the basic research that lays the groundwork for these clinical trials. Between 2010 and 2019, 356 drugs were approved by the FDA in the U.S., and NIH funding supported the development of 354 of them, totaling $187 billion.[5] In other words, nearly all, or 99.4%, of the drugs approved during that period trace their origins to NIH-supported research.

Given these high costs and failure rates, both the public and private sectors stand to benefit from technological advancements that can address common challenges in the development process. One such technology is artificial intelligence (AI), which can improve everything from study design and patient recruitment to real-time monitoring.

AI has already made strides in the early stages of drug discovery, helping researchers find potential disease targets and design new molecules.[6-7] Now, scientists are expanding AI's role in managing clinical trials, including writing

protocols, recruiting patients, and crunching trial data. The result is a faster, more cost-effective path from research to real-world application, potentially getting life-saving treatments to patients faster.

NUMBER OF AI/ML-RELATED CLINICAL TRIALS, 2013-2023

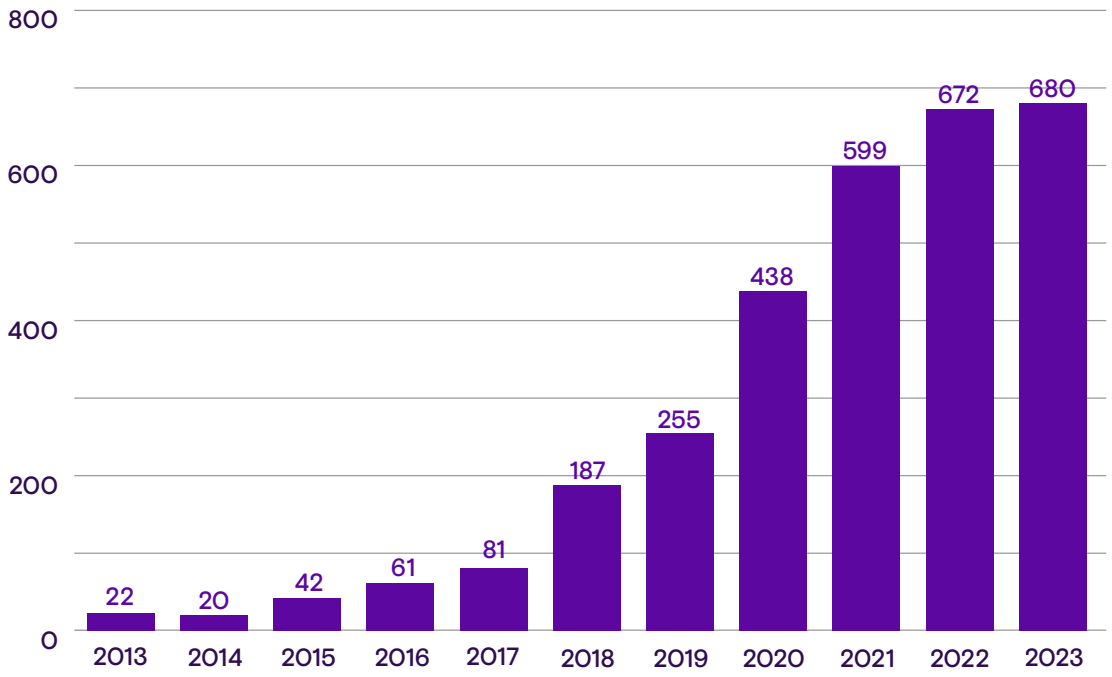


Fig. 2 Number of AI/ML studies registered on ClinicalTrials.gov from 2013 to 2023. n = 3,106. Source: Maru, Matthias, Kuwatsuru, & Simpson, 2024

As AI technologies gain traction in medical research, the clinical trial landscape is a key indicator of progress in the field. Data from ClinicalTrials.gov reveals a dramatic rise in the registration of AI/ML-driven studies, with numbers soaring from just 22 trials in 2013 to 680 in 2023.[8] The next decade will likely see heightened investment in these technologies with regulatory agencies like the FDA beginning to issue guidance on AI use in clinical trials.[9]

ClinicalTrials.gov also offers insight into how AI/ML is being used in clinical studies. Considering interventional trials, a majority were focused on diagnosis (28.2%) and treatment (24.4%), followed by 'other' purposes (12.5%) and prevention (8.2%).[8]

When looking only at randomized controlled trials (n=668), the focus shifted: Treatment (31.4%) came out on top, ahead of diagnostic (18.6%), with 'other' (11.1%) and prevention (10.9%) trailing behind.[8] Over time, the number of studies focused on diagnosis and treatment grew faster than those in other categories.

PURPOSE OF AI/ML-RELATED CLINICAL TRIALS

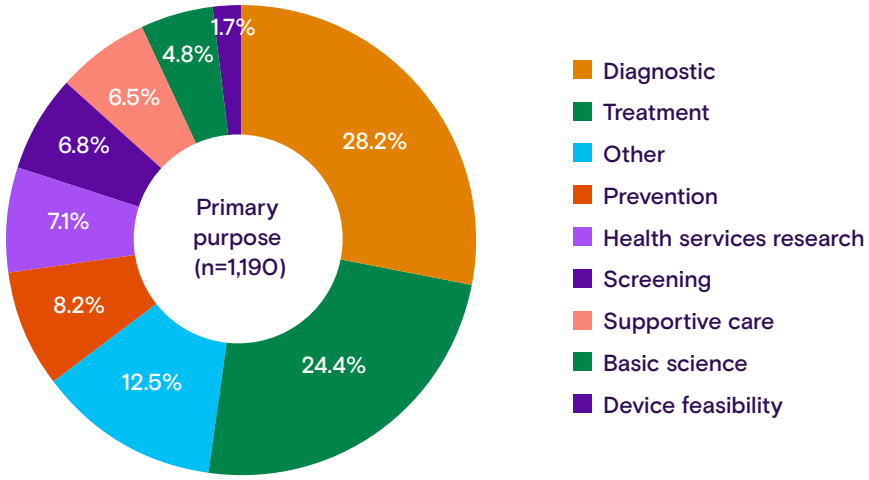


Fig. 3 Percentage of AI/ML studies registered on ClinicalTrials.gov from 2013 to 2023 by primary study purpose. Note: Primary study purpose was only available for interventional trials. Source: Maru, Matthias, Kuwatsuru, & Simpson, 2024

TOP 10 CLINICAL SPECIALTIES AND DISEASE AREAS IN AI/ML STUDIES

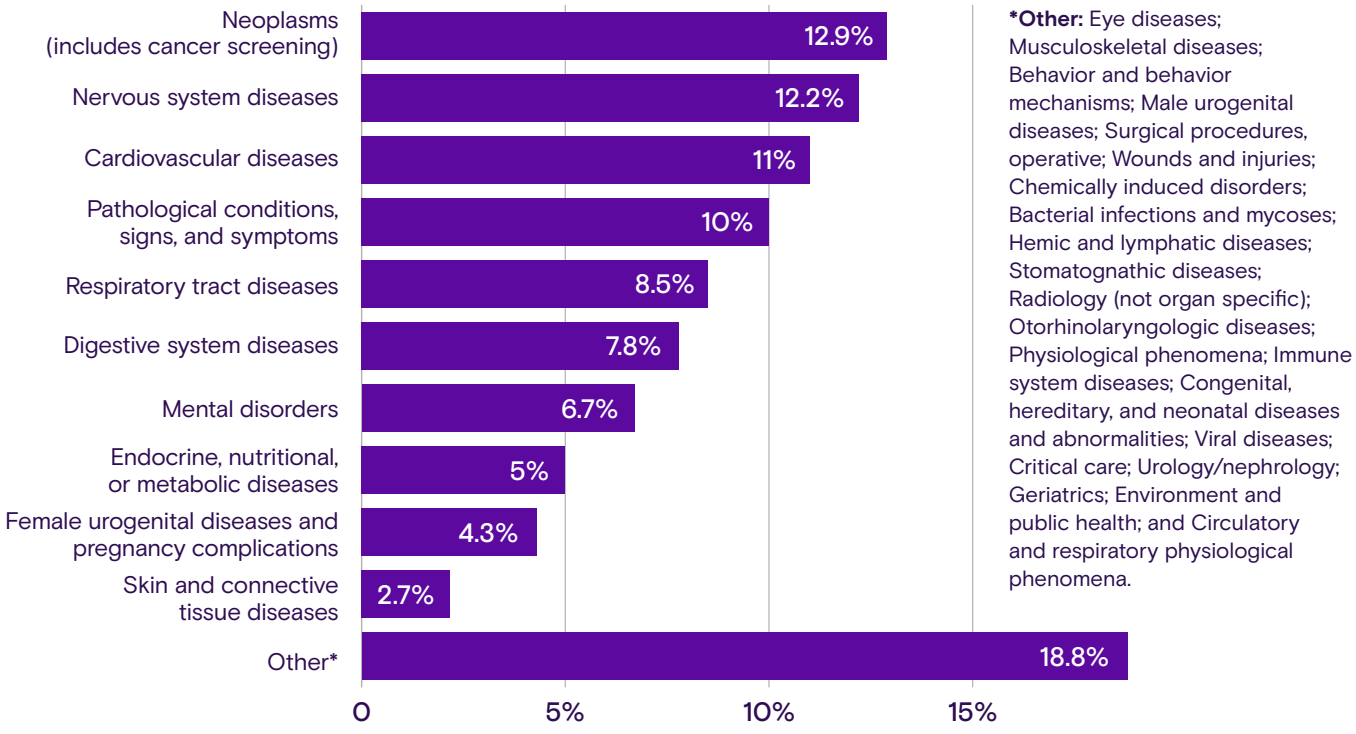


Fig. 4 Percentage of AI/ML studies registered on ClinicalTrials.gov from 2013 to 2023 by clinical specialties and disease areas. n=3,245. Source: Maru, Matthias, Kuwatsuru, & Simpson, 2024

The application of AI/ML spans a wide range of medical specialties; the top 10 clinical specialties and disease areas in AI/ML studies make up 81.1% of all clinical specialties. These include neoplasms (12.9%), nervous system diseases (12.2%), cardiovascular diseases (11%), pathological conditions, signs, and symptoms (10%), respiratory tract diseases (8.5%), digestive system diseases (7.8%), mental disorders (6.7%), endocrine, nutritional, or metabolic diseases (5%), female urogenital diseases and pregnancy complications (4.3%), and skin and connective tissue diseases (2.7%).[8]

The concentration of AI/ML studies in certain clinical specialties, such as oncology (neoplasms), suggests an effort to address high-burden diseases, with the potential for a significant impact on patient outcomes.

DISTRIBUTION OF LEAD SPONSORS BY SECTOR IN AI/ML STUDIES

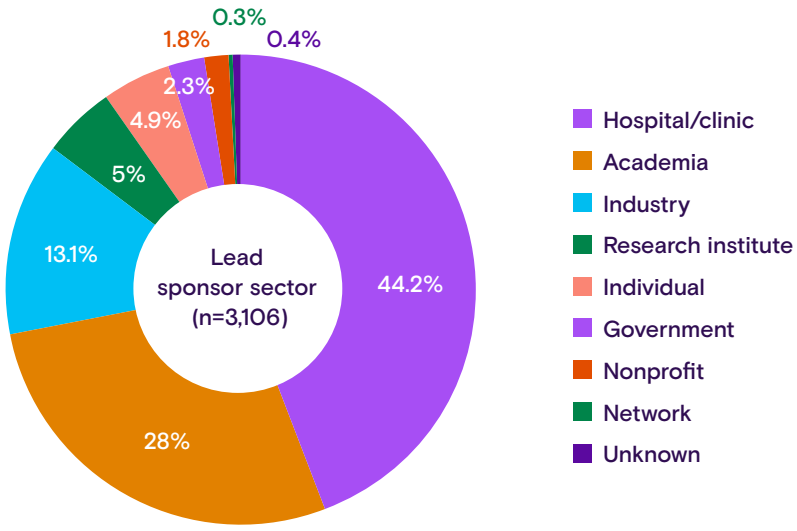


Fig. 5 Number of AI/ML studies registered on ClinicalTrials.gov from 2013 to 2023 by lead sponsor sector. Source: Maru, Matthias, Kuwatsuru, & Simpson, 2024

The wide application of AI/ML across medical specialties is matched by a diverse range of sponsors involved in these trials. A comparison of the original sector classifications from ClinicalTrials.gov with a reclassification based on sponsor names (n=3,106) shows some interesting trends.

Notably, there is a strong presence of hospitals/clinics (44.2%) and academic institutions (28%) alongside industry sponsors (13.1%).[8] The involvement of hospitals, clinics, and academic institutions in AI/ML trials has steadily increased since 2017, highlighting the growing collaboration between these sectors in advancing AI/ML-driven clinical research. For industry sponsors, this presents an opportunity to tap into a broader, more diverse ecosystem of expertise.

Implications for trial design, patient recruitment, and regulatory compliance

As AI continues to reshape the landscape of clinical trials, its impact on critical areas such as trial design and patient recruitment is becoming increasingly apparent. Equally important is the evolving relationship between AI-driven innovations and regulatory standards, as ensuring compliance with regulatory guidelines is required for the successful integration of AI into clinical trial processes.

STUDY DESIGN

Trial design—encompassing aspects such as drug dosage, sample size, and data collection methods—is a critical factor influencing the success of clinical studies. As trials become more complex, the integration of AI in refining study design becomes increasingly valuable. But how exactly does AI enhance this process?

> … AI-driven approaches are expected to enable more adaptive trial designs, allowing protocols to evolve in real-time …

AI-driven algorithms have been used to predict clinical trial outcomes, assess trial feasibility, and suggest study design refinements to increase the likelihood of success. One example is SEETrials, developed by Intelligent Medical Objects, which extracts safety and efficacy information from clinical trial abstracts (sourced using ChatGPT) to evaluate previous design frameworks and outcomes, enabling a more informed approach to future studies.

Looking ahead, AI-driven approaches are expected to enable more adaptive trial designs, allowing protocols to evolve in real-time based on emerging data, ultimately enhancing trial efficiency and reducing resource waste.

PARTICIPANT RECRUITMENT AND RETENTION

Building on its impact on trial design, AI can expedite the processes of both developing research eligibility criteria and finding eligible participants. A prime example is Stanford's Trial Pathfinder, which analyzes completed clinical trials to assess how adjusting participation criteria affects hazard ratios. These ratios compare the likelihood of negative outcomes (such as serious illness or death) between different patient groups. In lung cancer trials, Trial Pathfinder demonstrated that modifying eligibility criteria could double the number of eligible patients without increasing the hazard ratio, thereby expanding patient access while maintaining safety and trial integrity.[10]

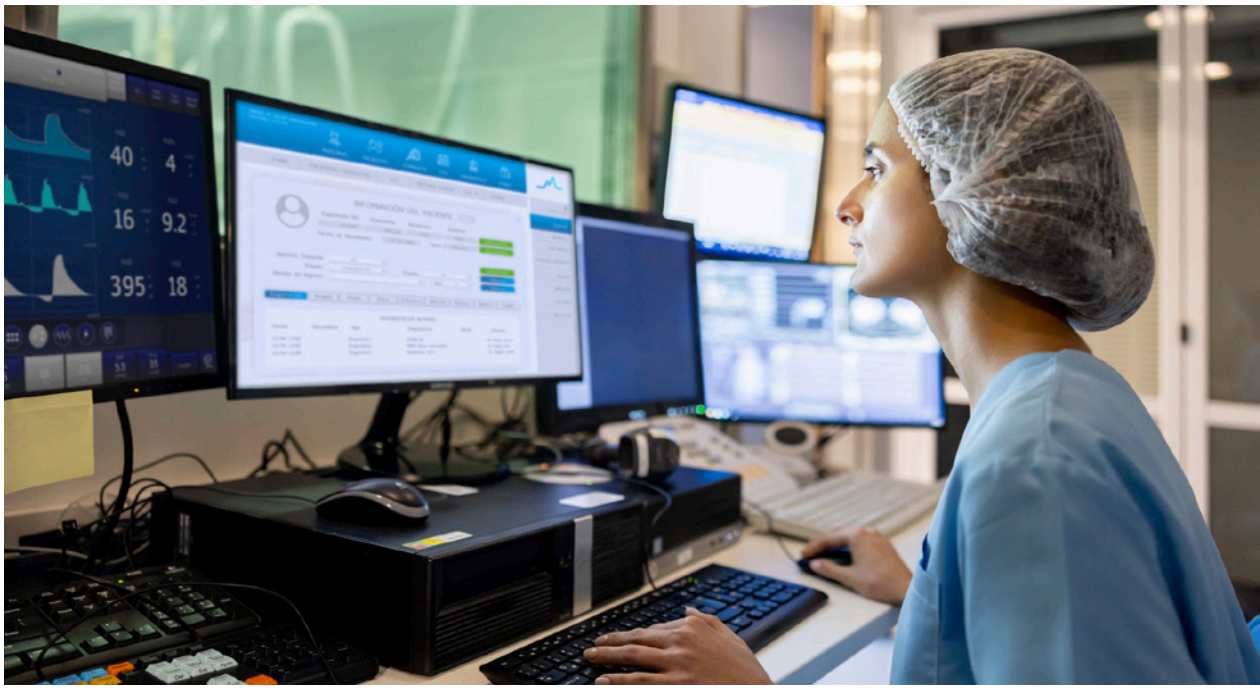
AI can also enhance retention, which is crucial for maintaining the statistical power and integrity of trial outcomes, by predicting which participants are at risk of attrition and offering tailored communication strategies to keep them engaged. For instance, AiCure's Patient Connect app leverages AI/ML to remotely monitor patient dosing behavior, sending automated reminders and alerts to reduce dropouts while also helping researchers anticipate attrition and ensure adherence to trial protocols.

REGULATORY COMPLIANCE

As AI-driven clinical trials gain traction, companies will need to establish and maintain strong relationships with regulatory bodies. This may involve working closely with regulators to ensure AI-driven methodologies are understood and accepted, and participating in industry-wide efforts to develop best practices for AI integration in clinical trials. Companies should also stay informed about upcoming changes in regulatory guidelines related to AI use in clinical research and actively engage in policy discussions.

In 2025, the FDA released draft guidance, Considerations for the Use of Artificial Intelligence to Support Regulatory Decision Making for Drug and Biological Products.[11] This guidance offers recommendations to the industry on the use of AI to produce information or data supporting regulatory decision-making regarding safety, effectiveness, or quality for drugs.

This marks a step in advancing AI integration in clinical trials and reinforces the importance of collaboration between companies and regulators to ensure that AI's role in clinical research is properly understood and validated.



Insight 2: The landscape of sites of service for surgical procedures is changing dramatically

It's well-known that ambulatory surgical centers (ASCs) are a growing force in American healthcare, and their influence is expected to increase, necessitating medical device companies to adapt their strategies to cater to the unique needs and dynamics of these centers.

From 2016 to 2023, the share of surgical procedures performed in hospitals dropped by 16%, according to Definitive Healthcare data. During the same period, ASCs saw their share of procedures rise by 24%. Factors driving this shift include advancements in surgical techniques and technologies, as well as evolving healthcare policies and reimbursement structures.

For example, CMS changes to the Inpatient-Only (IPO) list and the expansion of the ASC Covered Procedures List (CPL) have allowed more procedures to be performed in ASCs. The COVID-19 pandemic further accelerated this trend by pushing more procedures to outpatient settings due to hospital capacity constraints and safety concerns.

The chart below highlights these changes in procedural distribution between hospitals and ASCs.

SHARE OF SURGICAL PROCEDURES IN HOSPITALS & ASCS, 2016 - 2023

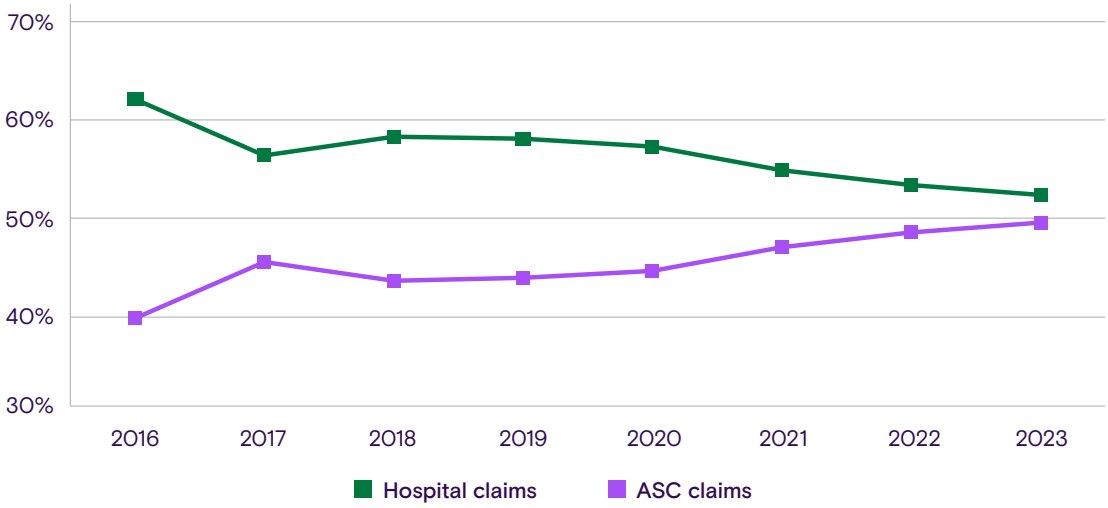
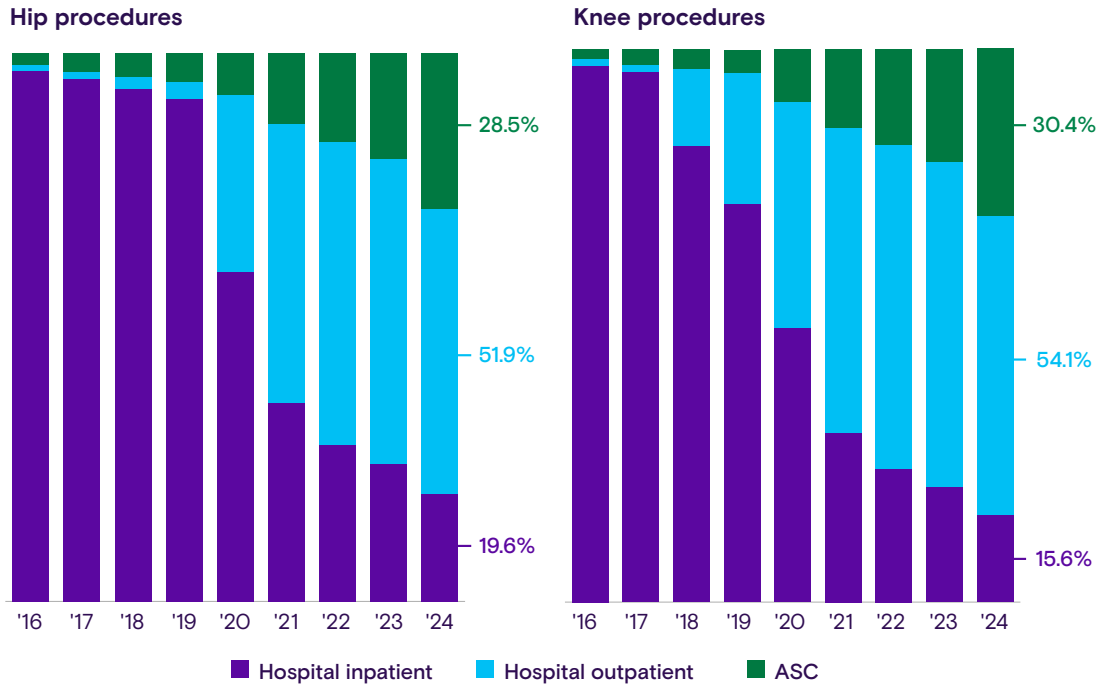


Fig. 6 Data from the Definitive Healthcare Atlas All-Payor Claims Dataset offers a broad, representative sample of U.S. healthcare but doesn't capture all procedures. This analysis includes hospital outpatient services identified by place of service and billing codes, and ASC activities with a surgical procedure code.

This shift is particularly noticeable in surgeries like joint replacements. From 2016 to 2024, the proportion of hip procedures performed in ASCs increased from 2.3% to 28.5%. These procedures include primary and revision hip arthroplasties, as well as hip arthrectomies. During the same period, hospital inpatient procedures for hip surgeries saw a sharp decline of 79.7%, while hospital outpatient procedures surged by 5,072%.

Knee procedures saw similar trends, with a significant increase in the share performed in ASCs from 2016 to 2024. Specifically, the proportion of knee procedures done in ASCs grew from 1.8% in 2016 to 30.4% in 2024. During this period, hospital inpatient procedures for knee surgeries declined by 83.9%, while hospital outpatient procedures saw a notable rise of 4,573%.

SHARE OF PROCEDURES BY SITE OF SERVICE, 2016-2024



Figs. 7 & 8 Data is from the Definitive Healthcare Atlas All-Payor Claims Dataset, which provides a broad, representative sample of U.S. healthcare data but does not capture all procedures.

As more procedures move to ASCs, medical device companies must recognize the unique operational and financial dynamics that differentiate ASCs from hospitals. ASCs are increasingly diverse, with both physician-owned and corporate-owned centers. Understanding factors driving purchasing and partnering decisions—such as ownership structures, vendor preferences, and affiliations with GPOs and IDNs—are all important for adapting sales strategies.

ASCs often have distinct priorities when selecting medical device partners, considering not only product cost but the broader value they bring. Providing strong clinical evidence on safety and outcomes, comprehensive training, and ongoing support can strengthen a company's value proposition, fostering long-term partnerships with ASCs.

Devices that improve quality metrics, such as PSI-90, offer a compelling example for hospital-affiliated ASCs struggling with CMS penalties. Additionally, integrated delivery networks and corporate entities may be particularly attracted to scalable solutions and broader network integration.

TOP 10 U.S. REGIONS WITH THE HIGHEST NUMBER OF ACTIVE ASCS, 2025

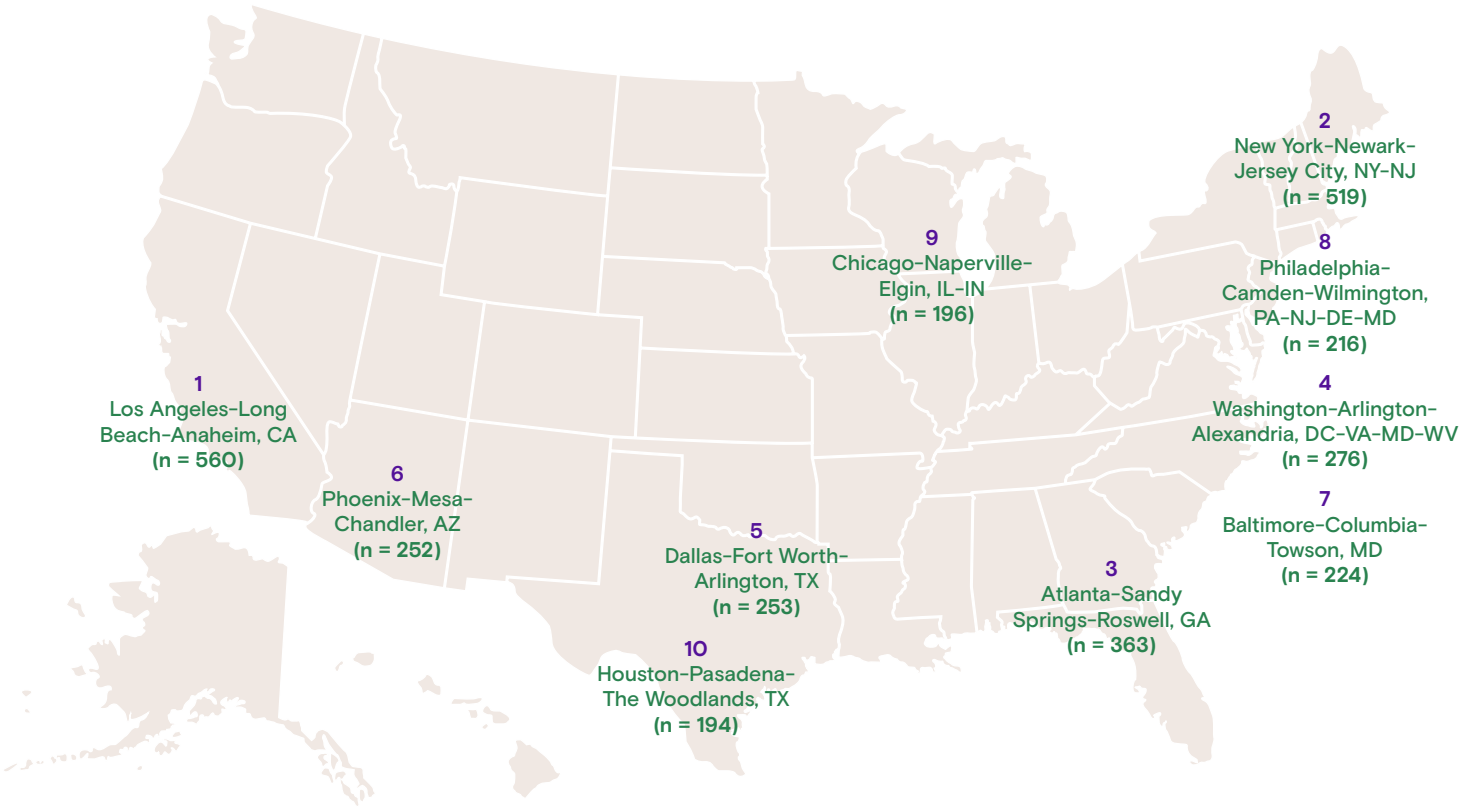


Fig. 9 Data is from the Definitive Healthcare Atlas All-Payor Claims Dataset, reflecting the number of active ambulatory surgery centers (ASCs) by core-based statistical area (CBSA).

Insight 3: Advances in gene editing are set to deliver a wave of new treatments for complex diseases

Fifty years after scientists first proposed modifying defective genes to treat genetic diseases, gene-editing technologies like CRISPR-Cas9 are now a reality. With several gene therapies already approved and many more in late-stage development, patients with rare, once untreatable conditions have new hope for effective treatment beyond traditional approaches.

The future of gene therapy will depend on striking a balance between fostering innovation and addressing the financial and logistical realities of healthcare systems, regulators, and the broader economy.

However, the journey from innovation to widespread adoption is still fraught with challenges. Developers must recover substantial research and development costs while ensuring profitability for investors. Payors are grappling with how to incorporate these expensive therapies into a healthcare system burdened by already high medical costs. Compounding these challenges, existing regulatory frameworks designed for more conventional drugs must be reworked to support the complexities of personalized gene-editing treatments.

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As of this writing, 46 different cell and gene therapies are licensed for use by the U.S. FDA.[12] One of the earliest, Provenge, a cell-based cancer immunotherapy for prostate cancer, was approved in 2010. More recent approvals highlight the rapid evolution of cell and gene therapies, with several groundbreaking treatments addressing critical unmet needs:

- Kymriah, developed by Novartis, marked the first FDA-approved CAR-T therapy, transforming the treatment of B-cell acute lymphoblastic leukemia and later expanding to other lymphomas.
- Casgevy and Lyfgenia are the first FDA-approved cell-based gene therapies for sickle cell disease (SCD), marking a major treatment milestone.

- Lenmeldy, developed by Orchard Therapeutics, provided a life-changing option for patients with metachromatic leukodystrophy (MLD), a rare and fatal neurodegenerative disease.
- Hemgenix, developed by CSL Behring, introduced the first gene therapy for hemophilia B, offering a one-time treatment designed to reduce or eliminate the need for ongoing factor IX infusions.

SELECTED LIST OF FDA-APPROVED CELLULAR AND GENE THERAPY PRODUCTS SINCE 2020

Therapy	Manufacturer	Indication	Year approved
Tecartus	Kite Pharma, Inc.	Mantle cell lymphoma	2020
Abecma	Celgene Corporation (Bristol-Myers Squibb)	Relapsed or refractory multiple myeloma	2021
Breyanzi	Juno Therapeutics (Bristol-Myers Squibb)	Large B-cell lymphoma, follicular lymphoma, mantle cell lymphoma	2021
Rethymic	Enzyvant Therapeutics GmbH	Pediatric T-cell immunodeficiency	2021
Stratagraft	Stratatech Corporation	Wounds	2021
Carvykti	Janssen Biotech, Inc.	Relapsed or refractory multiple myeloma	2022
Casgevy	Vertex Pharmaceuticals, Inc.	Beta-thalassemia, sickle cell disease	2022
Hemgenix	CSL Behring LLC	Hemophilia B	2022
Lantidra	CellTrans Inc.	Type 1 diabetes	2022
Lenmeldy	Orchard Therapeutics (Europe) Limited	Metachromatic leukodystrophy	2022
Omisirge	Gamida Cell Ltd.	Hematologic disorders	2022
Roctavian	BioMarin Pharmaceutical Inc.	Hemophilia A	2022
Skysona	bluebird bio, Inc.	Cerebral adrenoleukodystrophy	2022
Vyjuvek	Krystal Biotech, Inc.	Dystrophic epidermolysis bullosa	2022
Zynteglo	bluebird bio, Inc.	Beta-thalassemia	2022
Adstiladrin	Ferring Pharmaceuticals A/S	Bladder cancer	2023
Elevidys	Sarapeta Therapeutics, Inc.	Duchenne muscular dystrophy	2023
Lyfgenia	bluebird bio, Inc.	Sickle cell disease	2023
Amtagvi	Iovance Biotherapeutics, Inc.	Melanoma	2024
Beqvez	Pfizer, Inc.	Hemophilia B	2024
Tecelra	Adaptimmune LLC	Solid tumors	2024
Aucatzyl	Autolus Limited	Acute lymphoblastic leukemia	2024
Kebilidi	PTC Therapeutics	AADC deficiency	2024
Regenecyte	StemCyte, Inc.	HPC transplants for hematopoietic and immunologic reconstitution	2024
Ryoncil	Mesoblast, Inc.	Steroid-refractory acute graft versus host disease	2024
Symvess	Humacyte Global, Inc.	Vascular conduit for extremity arterial injury	2024
Encelto	Neurotech Pharmaceuticals, Inc.	Macular telangiectasia type 2	2025
Papzimeos	Precigen, Inc.	Recurrent respiratory papillomatosis	2025
Zevaskyn	Abeona Therapeutics, Inc.	Wounds in recessive dystrophic epidermolysis bullosa	2025

Fig. 10 A selected list of FDA-approved cellular and gene therapy products approved from 2020 through August 15, 2025. Source: U.S. Food and Drug Administration (FDA).

This progress is fueled by a dynamic ecosystem, encompassing established pharmaceutical leaders like Bristol-Myers Squibb and Novartis alongside pioneering biotech firms such as Bluebird Bio and Orchard Therapeutics.

The table to the right shows the distribution of FDA-approved cell and gene therapies across various therapeutic areas, illustrating the growing reach of these innovations. The most notable impacts are seen in hematology and oncology. Areas like neurology and dermatology are also being targeted.

Alongside the recent surge in FDA-approved therapies, funding trends provide a barometer of the field's momentum and future potential. From 2014 to 2023, venture capital and public funding in cell and gene therapies saw notable fluctuations, reflecting both increasing investor confidence and the sector's inherent challenges. Despite this, total funding grew from $1.03 billion in 2014 to $3.46 billion in 2023, indicating sustained long-term interest.

NUMBER OF FDA-APPROVED CELLULAR AND GENE THERAPY PRODUCTS BY THERAPY AREA

Therapeutic category	Products
Hematology	16
Oncology	13
Dermatology	5
Neurology	5
Immunology	2
Ophthalmology	2
Orthopedics	2
Endocrinology	1

Fig. 11 As of August 15, 2025. Source: U.S. Food and Drug Administration (FDA).

Investment is expected to slow in 2026 as venture capital shifts toward lower-risk technologies with clearer market pathways. High research and production costs, coupled with small patient populations, continue to challenge profitability. Still, the sector will likely attract investors and drugmakers who recognize its long-term potential.

CELL AND GENE THERAPY VENTURE CAPITAL AND PUBLIC FUNDING DEAL VALUE, 2014 – 2023

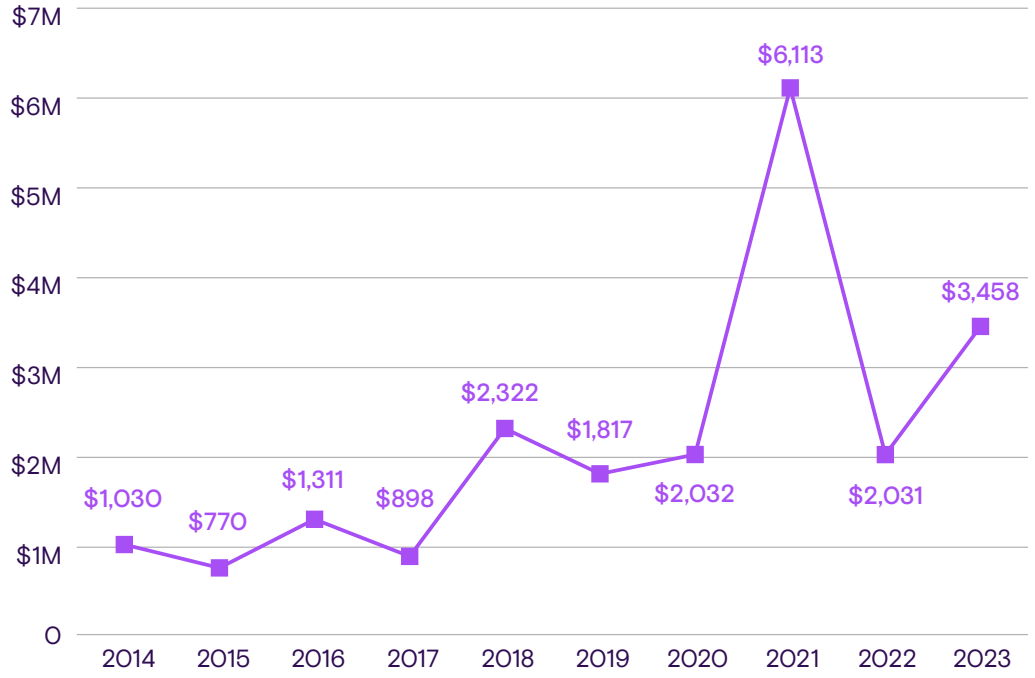


Fig. 12 Total deal value in millions of U.S. dollars. Excludes funding for companies only engaged in RNA therapeutics. Deals involve companies defined as life sciences companies. Source: Pitchbook

Implications for drug makers, pricing models, and market access

Advances in gene editing are set to drive a new wave of treatments for complex diseases, bringing the promise of personalized medicine closer to reality. However, realizing this potential comes with steep commercial hurdles, even as the number of therapies in pipelines and on the market begins to accelerate.

BALANCING UPFRONT COSTS WITH LONG-TERM VALUE

Gene-editing therapies are groundbreaking but come with extraordinarily high price tags. Casgevy, for example, is priced at $2.2 million for a single treatment, while Lenmeldy comes with a $4.25 million price tag, making them some of the most expensive drugs in the world.[13–14]

For conditions like sickle cell disease, which require lifelong management—including blood transfusions and hospitalizations—gene therapies could ultimately save the

healthcare system millions, especially in cases of expensive-to-treat diseases. But the multi-payor nature of the U.S. healthcare system complicates the financial equation. Given that patients often switch insurers throughout their lifetime, the entity covering the cost of a one-time curative therapy may not be the one reaping the long-term savings.

In contrast to the commercial market, Medicaid faces a unique set of challenges. Under the Medicaid Drug Rebate Program (MDRP), states are required to cover all FDA-approved drugs, with some exceptions, within established guidelines. The challenge to patient access in the U.S. lies in the ability of state Medicaid programs to allocate huge portions of their limited budgets to a small number of patients.

MANAGING COSTS THROUGH OUTCOMES-BASED PAYMENT MODELS

To address these financial complexities, manufacturers and payors are exploring outcome-based contracts, where payments for gene therapies are tied to specific clinical outcomes (e.g., sustained remission or a cure). These agreements can help align cost with value and mitigate financial risk for payors.

For instance, Bluebird Bio offers an outcomes-based contract alongside the upfront payment for its $2.8 million gene therapy, Zynteglo, reimbursing payors up to 80% of the cost if the patient does not achieve or maintain transfusion independence for two years post-treatment.[15]

Additionally, the U.S. federal government announced plans to implement a Cell and Gene Therapy Access Model in 2025, allowing state Medicaid agencies to negotiate outcomes-based payment arrangements with manufacturers. However, this policy was revoked on January 20, 2025, leaving the future of federal efforts to expand coverage for expensive cell and gene therapies uncertain.[16] If reinstated, the model could provide a major funding mechanism for gene therapies, particularly sickle cell disease, as an estimated 50-60% of people with SCD in the U.S. are enrolled in Medicaid.[17]

OVERCOMING BARRIERS TO ACCESS FOR PATIENTS

Gene therapies often target small, specific patient populations, and while therapies for conditions like beta-thalassemia, sickle cell disease, and hemophilia are emerging, payors impose strict access criteria. Factors considered in coverage decisions—such as patient age, disease severity, and administration challenges like a lack of expertise—can create barriers for patients.

Gene therapies typically involve complex, multi-step procedures that require specialized treatment centers and extended hospital stays, including cell collection, modification, intensive conditioning regimens, and prolonged recovery periods, all of which can deter patient uptake and restrict availability.

For example, despite recent approvals of Casgevy and Lyfgenia for sickle cell disease, only a small number of patients have started treatment, reflecting both the complexity of the process and ongoing access restrictions.[18] Adoption of Pfizer's Beqvez has also been sluggish, prompting the manufacturer to discontinue sales in early 2025, less than a year after the FDA approved the therapy for hemophilia B.

PAVING THE WAY FOR GENE THERAPY'S FULL POTENTIAL

For gene therapy to succeed, scientific breakthroughs alone won't be enough. Developers will need thoughtful solutions to address the financial constraints, regulatory challenges, and operational complexities that continue to impede successful commercialization.

Companies will want to consider the patient journey, the competitive landscape, the provider ecosystem, and the changing payor and regulatory environments to support the long-term viability of gene-based treatments. With continued collaboration and strategic planning across stakeholders, gene therapy can overcome current obstacles and deliver life-changing treatments for patients in need.

Insight 4: A new administration brings policy shifts that could impact regulatory processes, requiring proactive planning

Each new presidential administration brings shifts in health priorities and policies that can shape the life sciences sector. These changes, driven by executive orders, policy directives, and agency guidance, can influence drug approvals, compliance expectations, and the overall direction of public health and scientific innovation.

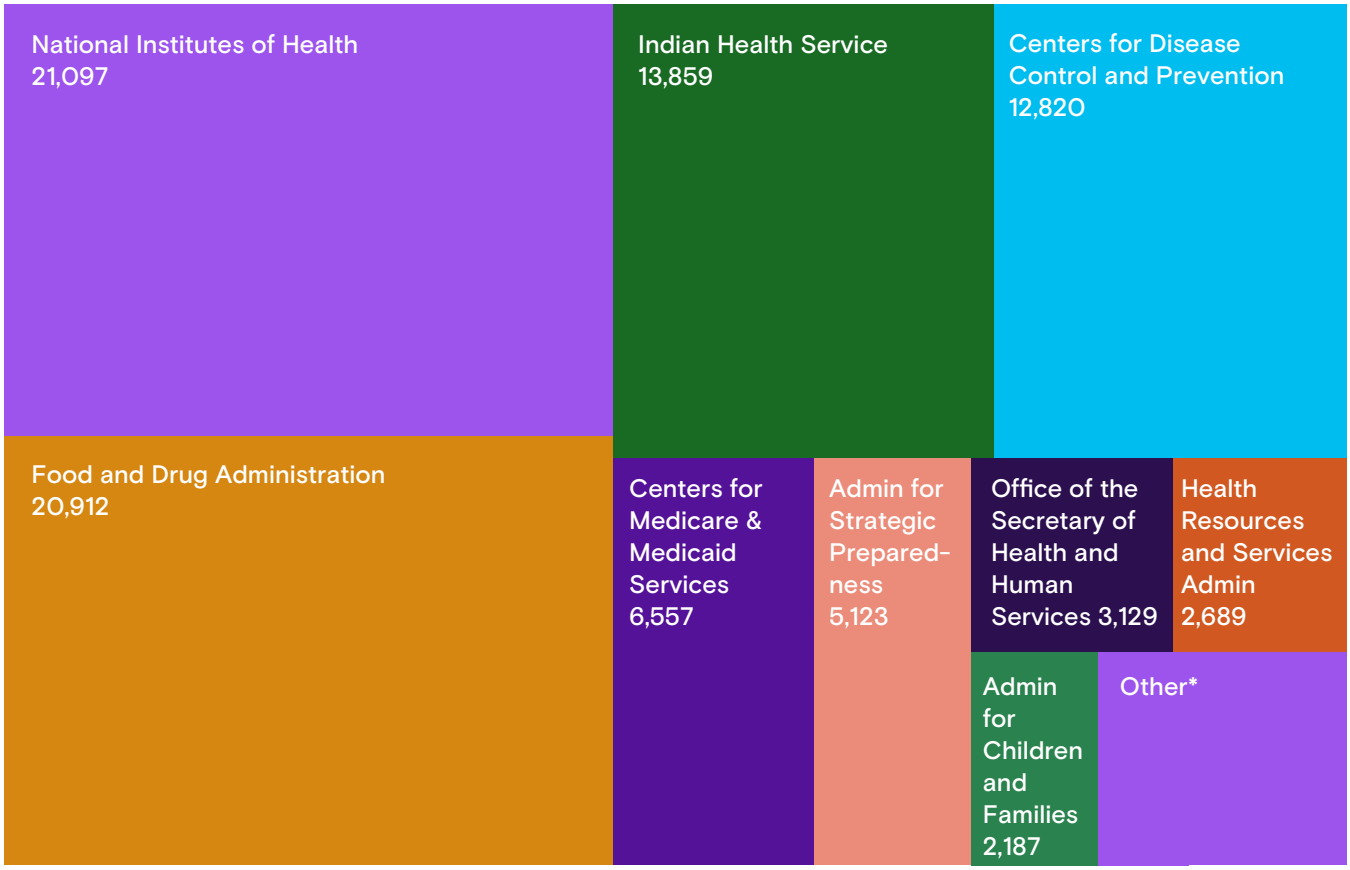
Since January 2025, the Trump administration has moved quickly to establish its priorities and set the tone for its direction. Recent federal actions, such as terminating leases for FDA facilities, laying off employees, and canceling NIH grants, have created a climate of uncertainty.[19-21] The later reversal of some of these decisions adds to the instability in key agencies that support the life sciences sector, further complicating the operating environment for companies.[22]

Examining recent data on the federal workforce within key agencies like the FDA and funding allocations to the NIH can provide insights into how staffing changes and resource constraints may affect regulatory processes and therapeutic development.

As of late 2024, the Department of Health and Human Services (HHS) employed more than 91,000 people. Most of these employees work for the NIH, which conducts medical research and funds health studies, followed by the Food and Drug Administration (FDA), which ensures the safety and efficacy of food, drugs, and medical devices.

Other significant agencies include the Indian Health Service (IHS), which provides healthcare to Native American populations, the Centers for Disease Control and Prevention (CDC), which focuses on public health and disease prevention, and the Centers for Medicare and Medicaid Services (CMS), which administers national healthcare programs like Medicare and Medicaid.

WHERE DEPARTMENT OF HEALTH AND HUMAN SERVICES EMPLOYEES WORK



Other:

Office of Inspector General	1,544
Substance Abuse and Mental Health Services Administration	916
Office of Medicare Hearings and Appeals	662
Program Support Center	410
Agency for Healthcare Research and Quality	298
Administration for Community Living	243
Agency for Toxic Substances and Disease Registry	173

Fig. 13 Federal civilian workforce at executive departments and major agencies, September 2024. Source: Definitive Healthcare analysis of Office of Personnel Management data.

Since early 2025, the Trump administration, through the Department of Government Efficiency (DOGE), has implemented workforce reductions across federal agencies, including HHS. As of August, more than 15,800 employees within HHS have been laid off, affecting the CDC, the FDA, and NIH. Plans remain in motion to reduce the HHS workforce by a total of 20,000 jobs.[23]

At the FDA, dismissals included staff reviewing drug and device submissions, many of whom were funded by industry-paid user fees, causing ongoing delays in the approval process for manufacturers.[24] In response, the FDA moved to rehire some FDA staffers affected by the cuts.[25] However, following further administrative decisions, many of those rehired were subsequently laid off again, exacerbating uncertainty for the agency.

WHERE NIH FUNDING IS ALLOCATED, 2023

NIH division, institute, or center	Funding*
National Cancer Institute (NCI)	$7,320,159
National Institute of Allergy and Infectious Diseases (NIAID)	$6,562,279
National Institute on Aging (NIA)	$4,407,623
National Heart, Lung, and Blood Institute (NHLBI)	$3,982,345
National Institute of General Medical Sciences (NIGMS)	$3,239,679
Office of the Director (OD)	$3,074,514
National Institute of Neurological Disorders and Stroke (NINDS)	$2,813,925
National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)	$2,442,171
National Institute of Mental Health (NIMH)	$2,337,843
Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)	$1,749,078
National Institute on Drug Abuse (NIDA)	$1,662,695
National Institute of Environmental Health Sciences (NIEHS)	$997,014
National Center for Advancing Translational Sciences (NCATS)	$923,323
National Eye Institute (NEI)	$896,549
National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)	$685,465
National Human Genome Research Institute (NHGRI)	$663,200
National Institute on Alcohol Abuse and Alcoholism (NIAAA)	$595,318
National Institute on Deafness and Other Communication Disorders (NIDCD)	$534,333
National Institute on Minority Health and Health Disparities (NIMHD)	$524,395
National Institute of Dental and Craniofacial Research (NIDCR)	$520,163
National Library of Medicine (NLM)	$497,548
National Institute of Biomedical Imaging and Bioengineering (NIBIB)	$440,627
Office of Budget and Finance (B&F)	$350,000
National Institute of Nursing Research (NINR)	$197,693
National Center for Complementary and Alternative Medicine (NCCAM)	$170,384
Fogarty International Center (FIC)	$95,162

Fig. 14 *In thousands of dollars. NIH appropriations by institute, division, or center in 2023.
Source: Definitive Healthcare analysis of NIH Almanac data.

About half of the FDA's budget, around $3.3 billion, comes from user fees, which are paid by drugmakers, device manufacturers, and tobacco companies.[26]

While workforce reductions across HHS have had immediate effects on agencies like the FDA, the broader implications of funding shifts within the NIH are also worth considering. As the world's largest public funder of biomedical research, the NIH has long played a critical role in de-risking early-stage research and catalyzing private investment.[27]

Reductions in NIH funding could have downstream effects on life science companies, particularly for startups and early-stage firms that rely on federal grants and partnerships with research institutes. Clinical trials conducted at NIH-funded sites may also face disruptions, potentially affecting development timelines.

Given that foundational breakthroughs—including mRNA vaccines, gene therapies, and targeted cancer treatments—emerged from NIH-backed research, ongoing funding uncertainties underscore the importance of ensuring sustained support to drive future advancements in the industry.

Navigating uncertainty with proactive planning

Life science companies should closely monitor these developments and strategically adapt to mitigate potential disruptions. Strengthening private investment channels, diversifying funding sources, and fostering industry partnerships will be critical to sustaining early-stage research. A proactive approach to R&D planning—anticipating potential delays and aligning development strategies accordingly—will help maintain momentum and ensure continued innovation in a shifting financial landscape.

Recent workforce reductions at the FDA and uncertainty surrounding regulatory processes will likewise require companies to remain flexible and develop contingency plans for potential disruptions. While the full impact of FDA layoffs is still unfolding, signs of strain are already visible, including growing review backlogs and missed approval deadlines. If staffing cuts persist or worsen, delays in product reviews, enforcement actions, and other core agency functions are likely to escalate.

Given the potential for these disruptions, companies must proactively plan for delays, especially those with upcoming PDUFA or MDUFA dates. Although user fee commitments are meant to ensure timely reviews, understaffing could still present challenges. Companies should engage in scenario planning, maintain close communication with the FDA, and prepare for various scenarios to mitigate risks and ensure a smoother path forward in 2026.

Charting the path forward

The life sciences industry is charging ahead with groundbreaking innovations—from AI-driven clinical trials to gene editing breakthroughs—but the road ahead isn't without twists. Shifting care settings, evolving regulations, and policy shake-ups could all impact how quickly these advances reach patients. Success in this landscape takes more than innovation—it will require companies to anticipate and adapt to ongoing shifts and maintain a focus on how market dynamics and regulatory priorities evolve to ensure innovations translate into real-world impact. With the right data and insights, life sciences companies can ensure their breakthroughs reach the people who need them most.

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