

HOT INDICATIONS LIST 2021 HOT MOAs

**A global analysis
of pharmaceutical
investment intensity
across R&D programs**

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AS PREDICTED, THE HEALTHCARE INDUSTRY HAS SEEN RAPID CHANGES AND UNPRECEDENTED GROWTH IN THE LAST YEAR DUE TO THE COVID-19 PANDEMIC.

While the world around us changed, pharma and biotech companies innovated and inspired, springing into action with COVID vaccines and treatments in response to the pandemic. After just under nine months following the WHO's pandemic declaration, the UK MHRA approved the first major COVID vaccine, made by Pfizer. In the face of the variety of challenges the pandemic posed, pharma and biotech companies were resilient. Existing clinical development plans were adapted to account for any changes in trials to ensure new treatments for other diseases could move forward to help patients in need.

Despite this need to adapt, the FDA approved 53 novel drugs in 2020, the second-highest number of approvals in the last twenty years. In Kx's seventh annual analysis of global R&D investments, our experts identified the top indications for industry focus across 20 therapeutic areas (TAs).

Top Indications & Therapeutic Areas 2021

On par with the last several years, Oncology was the leading TA overall, with 12,125 companies running 8,820 Phase II and Phase III clinical trials. Oncology has led the other TAs in all categories since 2015, highlighting continued interest and investment in finding more effective cancer treatments. The top 11 indications this year were all cancers, including **breast cancer (#1)**, which remained at the top of the list. With a 25% increase in investment in 2020, this indication continues to drive innovation. Antibody-drug conjugates were hotly anticipated launches this year, after the late 2019 FDA approval of AstraZeneca and Daiichi Sankyo's Enhertu, an antibody-drug conjugate combining a HER2 antibody with a topoisomerase I inhibitor, for metastatic or unresectable HER2-positive breast cancer.¹ Enhertu was also approved for **gastric cancer (#11)** in January 2021. Gilead's Trodely, combining a first-in-class monoclonal antibody that targets the TROP2 protein with another topoisomerase I inhibitor, won accelerated FDA approval for triple-negative breast cancer in April 2020, based on Phase II data, followed by fulfillment of post-marketing requirements in April 2021.² In 2019, Novartis launched their first-in-class PI3K inhibitor for breast cancer treatment, Piqray; Roche launched a companion diagnostic in late 2020, the cobas PIK3CA Mutation Test, which detects PIK3CA mutations in patients with breast cancer to help determine whether Piqray would be a suitable treatment option.³ We predict Oncology, particularly breast cancer, will continue to be an area of significant investment in the future.

Several of last year's top Neurology indications dropped in ranking, notably **Parkinson's disease (#24)** and **Alzheimer's disease (AD, #19)**. AD fell from sixth, with total IPO and VC investment dropping from \$1.1B to \$467M. This decrease in funding and the decline in the number of programs in development led to the drop in this year's rankings, which we predicted in last year's [Neurology Deep Dive](#). However, the FDA's 2021 accelerated approval of Biogen's Aduhelm has revitalized interest and sparked investments in both approved and in-development treatments, like Eli Lilly's donanemab. We anticipate seeing AD-related investment rise in reaction to this development, especially tau protein antibodies. Roche and AC Immune's Phase II anti-tau antibody semorinemab recently showed the first clinical trial evidence of a tau-targeting monoclonal antibody benefitting patients with mild to moderate Alzheimer's disease, reducing cognitive decline (ADAS-Cog11) by 43.6% compared to placebo.⁴ TauRx's TRx0237 is the most advanced treatment in the tau pipeline, in a Phase III trial that will be completed in 2022.⁵

Hottest MOAs for Notable Indications

BREAST CANCER:
Cyclin-dependent kinase (CDK) inhibitors

PARKINSON'S:
Alpha-synuclein

ALZHEIMER'S:
Tau protein agonists

NASH:
Farnesoid X receptor (FXR) agonists

¹ https://www.breastcancer.org/treatment/targeted_therapies/enhertu

² <https://www.gilead.com/news-and-press/press-room/press-releases/2021/4/fda-approves-trodely-the-first-treatment-for-metastatic-triplenegative-breast-cancer-shown-to-improve-progression-free-survival-and-overall-survival>

³ <https://www.fiercebiotech.com/medtech/roche-launches-new-breast-cancer-test-seeking-out-pik3ca-mutations>

⁴ <https://www.alzforum.org/news/research-news/first-cognitive-signal-tau-immunotherapy-works>

⁵ <https://clinicaltrials.gov/ct2/show/NCT03446001?term=NCT03446001&draw=2&rank=1>

HOT MOAs

While the top seven TAs remain the same, Hepatology broke into the top ten, ranking eight this year. Within Hepatology, Non-alcoholic steatohepatitis (**NASH, #27**) is the top-ranked indication. Interest in NASH is driven by innovative therapies like CytoDyn’s Vyrologix, a Phase II C-C chemokine receptor type 5 (CCR5) antibody, and Bristol-Myers Squibb and Ambrx’s Pegbelfermin, a Phase II fibroblast growth factor 21 (FGF21) stimulant. Various programs with MOAs discussed in this analysis are also in clinical trials for NASH, including AXL inhibitors.

TA Investment Intensity

THERAPEUTIC AREA CATEGORY	2021 RANK	2020 RANK
ONCOLOGY	1	1
INFECTIOUS DISEASE	2	2
NEUROLOGY	3	3
ENDOCRINOLOGY	4	4
OPHTHALMOLOGY	5	5
IMMUNOLOGY	6	6
DERMATOLOGY	7	7
HEPATOLOGY	8	11
HEMATOLOGY	9	8
PULMONOLOGY	10	13
GASTROENTEROLOGY	11	9
PSYCHIATRY	12	15
MUSCULOSKELETAL	13	10
CARDIOVASCULAR	14	14
NEPHROLOGY	15	16
UROLOGY & WOMEN'S HEALTH	16	12
OTHER	17	18
INFLAMMATORY	18	17
TRANSPLANT	19	20
ENT	20	19

Top Movers 2021

There were several dramatic jumps within the Hot Indications list this year. One of the most significant leaps was **essential tremor (#157)**, which moved up 348 places from last year's analysis. There was new investment in the indication; Cadent Therapeutics raised \$15M for its Phase II trial of CAD-1883 and was subsequently acquired by Novartis; Praxis Medicines raised \$14M for its two late-stage tremor small molecule treatments.⁶ Acquisitions, partnerships, and major players in this space suggest the potential for sustained interest. Jazz Pharmaceuticals' JZP-385, a highly selective modulator of T-type calcium channels, is planned for a Phase IIb clinical trial. In November 2020, Biogen and Sage Therapeutics announced their agreement to jointly develop SAGE-324, which recently completed its Phase II clinical trial for essential tremor, as well as other neurological disorders.⁷

Leber's hereditary optic neuropathy (LHON, #169) rose 334 places, despite its rarity; the disease only affects 1 in 50,000 people.⁸ The indication saw an increase in both companies and clinical trials. In 2020, Neurophth Therapeutics' Phase III gene therapy NR082 was granted an orphan drug designation by the FDA, reflecting the significant interest in finding a cure for this rare disease. In June 2021, GenSight Biologics' gene therapy LUMEVOQ was granted a Cohort ATU by the French regulator (ANSM).⁹ In September 2021, it was granted Promising Innovative Medicine (PIM) designation by the UK's MHRA; the European Medicines Agency is expected to make a decision in 2022.¹⁰ The attention surrounding LHON reflects the ongoing interest in gene therapies, including for eye diseases, which present significant challenges with drug delivery.

Several indications saw dramatic jumps in 2021, including the first-ever appearance of a new indication in the top 20 list.

⁶ <https://praxismedicines.com/>

⁷ <https://www.businesswire.com/news/home/20201127005340/en/Biogen-and-Sage-Therapeutics-Announce-Global-Collaboration-to-Develop-and-Commercialize-Potential-Breakthrough-Therapies-in-Depression-and-Movement-Disorders>

⁸ <https://rarediseases.org/rare-diseases/leber-hereditary-optic-neuropathy/>

⁹ <https://www.gensight-biologics.com/2021/07/05/gensight-biologics-announces-approval-of-the-lumevoq-cohort-temporary-authorization-for-use-atuc-in-france/>

¹⁰ <https://www.gensight-biologics.com/2021/09/06/uk-mhra-grants-gensight-biologics-lumevoq-ophthalmic-gene-therapy-promising-innovative-medicine-designation/>

The Impact of COVID-19

Unsurprisingly, **COVID-19 (#12)** was the first indication to break into the top 20 on the Hot Indications List in its first year. In 2020, 699 companies worldwide pursued therapies and vaccines for COVID-19 via 1,014 clinical trials. IPOs and VCs invested \$3.3B in 2020, the second-largest combined investment in any indication; only **non-small cell lung cancer (NSCLC, #2)** raised more, at \$3.8B.

As predicted in [2020's Hot Indications analysis](#), COVID-19 had wide-ranging implications for the healthcare industry, especially on the Infectious Disease TA. Investment in Infectious Disease jumped dramatically, with increases of more than \$13M in VC funding and \$800k in funds raised by IPOs compared to 2019. **Influenza (#13)** jumped in rank from 42 last year, likely the result of preparation for the next pandemic. Vaccine approaches for COVID-19 are now also being used for flu vaccines, with Moderna developing an mRNA-based flu vaccine that is currently in a Phase I/II study.¹¹ Between the CDC purchasing 14 times the flu vaccine doses they typically have for adults in 2020 and major players like Sanofi Pasteur and Seqirus committing to increasing their dose production last year, the pandemic played a role in influenza investment and interest.¹² In other TAs, paused clinical trials and delays in regulatory approval due to COVID-19 have slowed pipelines, but not stopped drug development.

¹¹ <https://investors.modernatx.com/news-releases/news-release-details/moderna-announces-first-participant-dosed-phase-12-study-its>

¹² <https://www.washingtonpost.com/health/2020/06/24/cdc-drugmakers-boost-flu-vaccine-doses-amid-fears-an-unprecedented-respiratory-illness-season/>

Hot MOAs

Our Hot Indications analysis has focused on investments into specific indications and their respective therapeutic areas in past years. This year, we dove into mechanisms of action (MOAs), assessing the trends in development across multiple indications and TAs. Of the 2,335 MOAs we evaluated, three offer particularly promising advances:

- **PHOSPHATIDYLINOSITOL 3-KINASE (PI3K) INHIBITORS**
- **AXL INHIBITORS**
- **STEM CELL STIMULANTS**

Phosphatidylinositol 3-Kinase (PI3K) Inhibitors

PI3K inhibitors act on one or more PI3-kinase enzymes, part of the critical PI3K/AKT/mTOR pathway that transmits signals driving many cellular functions, including growth control, metabolism, and translation inhibition.

PI3K inhibitors have been primarily developed as cancer treatments; translation inhibition is theorized to result in tumor suppression. Novartis' Piqray, one of two drugs that the US FDA has approved, induced tumor shrinkage in three of four patients with a PIK3CA mutation during its pivotal trial.¹³ TG Therapeutics's U2 and MEI Pharma's ME-401, both in development for **chronic lymphocytic leukemia (CLL, #45)** and **non-Hodgkin lymphoma (NHL, #10)**, are currently in Phase III.

PI3K inhibitors are among the many MOAs proposed to treat symptoms of **COVID-19 (#12)**. This MOA's ability to suppress inflammation may allow patients to recover faster.¹⁴ Similarly, PI3K inhibitors are also being investigated for inflammatory respiratory disease, likely due to the overlap across similarly targeted pathways.

INDICATIONS AND THIS MOA



IMPLICATED IN THE FOLLOWING INDICATIONS AND TAs, AMONG OTHERS:

Infectious Disease: **COVID-19**
 Neurology: **Parkinson's disease**
 Oncology: **Breast cancer, Colorectal cancer**
 Pulmonology: **Pulmonary hypertension**



BY THE NUMBERS:

~100
programs in Phase II

~72
programs in Phase I

¹³ <https://www.hcp.novartis.com/products/piqray/metastatic-breast-cancer/efficacy/>

¹⁴ <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7417114/>

AXL Inhibitors

AXL is a receptor tyrosine kinase involved in the stimulation of cell proliferation and survival. Multiple signaling pathways are activated downstream of AXL, including PI3K-AKT-mTOR, MEK-ERK, NF- κ B, and JAK/STAT. These pathways are indicated in several Oncology indications, like **breast cancer (#1)** and **NSCLC (#2)**, as well as indications in other TAs, including **epilepsy (#58)**, **COVID-19 (#12)**, and **dermatitis (#38)**.

While several drugs classified as AXL inhibitors are on the market, many target multiple kinase receptors in addition to AXL, such as Exelixis' Cabometyx/Cometriq. The latest-stage AXL selective inhibitor in the pipeline is BerGenBio and Rigel Pharmaceuticals' bemcentinib, an oral small molecule currently in Phase II clinical trials for multiple top 10 indications, including **NSCLC (#2)** and **acute myeloid leukemia (AML, #9)**. Like many other AXL inhibitors, bemcentinib is also being investigated as a combination therapy with existing and emerging immunotherapies and chemotherapy.

Similar to PI3K inhibitors, the initial focus of AXL inhibitors was Oncology. Both classes target key pathways and are therefore of interest for multiple cancers. However, as research reveals the molecular role of AXL, inhibitors are expanding into other indications, including bemcentinib for **COVID-19 (#12)** and **NASH (#27)**.

Recently, the AXL pathway was found to be active in chronic fibrotic diseases in several organs, including the liver and intestines.¹⁵ As a result, AXL inhibitors could be an effective approach to help those with intestinal fibrosis, such as **Crohn's disease (#44)**.¹⁶ AXL may also play an important role in **Zika virus (#325)**, with several studies demonstrating that AXL allows entry of the virus into host cells. However, other studies have provided conflicting results.¹⁷ Further research may increase investment and attention for this MOA for those emerging treatments.

INDICATIONS AND THIS MOA



IMPLICATED IN THE FOLLOWING INDICATIONS AND TAS, AMONG OTHERS:

Hepatology: **Non-alcoholic steatohepatitis**

Infectious Disease: **COVID-19, Zika virus**

Oncology: **Chronic lymphocytic leukemia, Melanoma, Non-Hodgkin lymphoma, Non-small cell lung cancer**

Nephrology: **IgA nephropathy**

Pulmonology: **Pulmonary fibrosis**



BY THE NUMBERS:

~102
programs in Phase II

~32
programs in Phase I

¹⁵ <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6834320/>

¹⁶ <https://academic.oup.com/ibdjournal/article/27/3/303/5872811>

¹⁷ <https://www.frontiersin.org/articles/10.3389/fcimb.2021.575346/full>

Stem Cell Stimulant

Stem cells mediate cell repair by acting as an anti-inflammatory agent, supporting tissue reformation, and recruiting other necessary cells for tissue growth. Due to the relatively low number of MHC molecules on their surface, stem cells have also been shown to have low immunogenicity. In addition, they have been found to secrete chemokines, which alter immune response and promote tolerance of the new tissue. These factors make this MOA ideal for a variety of indications, particularly in regenerative medicine.

Stem cells were initially used for transplants and continue to be used for grafts, like Smith & Nephew's Grafix, a mesenchymal cell therapy, which is in the market to treat chronic wounds. This MOA has expanded to support treatments for a large range of indications, including **heart failure (#49)**, **osteoarthritis (#99)**, and **Parkinson's disease (#24)**. Capricor Therapeutics' CAP-1002, a Phase II cardiac stem cell therapy, is focused on heart cell regeneration.¹⁸ International Stem Cell Corporation's ISC-hpNSC, a neural stem cell therapy, is currently in Phase I for **Parkinson's disease (#24)** and pre-clinical development for **traumatic brain injury (#147)**.

With this growth in stem cell stimulant treatments, not every treatment has been a success. Mesoblast's Revascor, a mesenchymal cell therapy for **heart failure (#49)**, failed to reach its Phase III primary endpoint of reducing hospitalizations in advanced chronic heart failure patients.¹⁹ The FDA recommended that Brainstorm Cell Therapeutics generate more clinical data for Phase III NurOwn Program One, a mesenchymal bone marrow stromal cell therapy, for **amyotrophic lateral sclerosis (ALS, #57)**. The clinical data provided did not meet the FDA's threshold of substantial evidence.²⁰ NurOwn Program One is also in Phase II for **multiple sclerosis (MS, #34)**. Given the potential of this MOA to treat life-altering diseases and the current development intensity we observe, we anticipate continued interest despite clinical trial failures.

INDICATIONS AND THIS MOA



IMPLICATED IN THE FOLLOWING INDICATIONS AND TAS, AMONG OTHERS:

Cardiovascular: **Buerger's disease, Heart failure**
 Dermatology: **Dermal ulcers, Psoriasis**
 Gastroenterology: **Crohn's disease, Gastrointestinal fistula**
 Hematology: **Avascular necrosis, Sickle cell disease**
 Immunology: **Osteoarthritis, Rheumatoid arthritis**
 Infectious Disease: **COVID-19**
 Nephrology: **Chronic kidney disease**
 Neurology: **Alzheimer's disease, Amyotrophic lateral sclerosis, Cerebral palsy, Pain, Parkinson's disease**
 Oncology: **Head & neck cancers, Melanoma**
 Pulmonology: **Chronic obstructive pulmonary disease**
 Transplant: **Organ and tissue transplant**



BY THE NUMBERS:

~202
programs in Phase II

~90
programs in Phase I

¹⁸ <https://capricor.com/product-pipeline/cap-1002/>

¹⁹ <https://www.bioworld.com/articles/501375-mesoblasts-revascor-fails-to-meet-primary-endpoint-in-phase-iii-chronic-heart-failure-trial>

²⁰ <https://www.prnewswire.com/news-releases/brainstorm-announces-high-level-fda-feedback-on-nurown-als-clinical-development-program-301232436.html>

Top 100 Hot Indications

2021 RANK	INDICATION	THERAPEUTIC AREA	2020 RANK
1	Breast Cancer	Oncology	1
2	Non-small Cell Lung Cancer (NSCLC)	Oncology	3
3	Pancreatic Cancer	Oncology	7
4	Head & Neck Cancers	Oncology	14
5	Prostate Cancer	Oncology	9
6	Colorectal Cancer	Oncology	5
7	Melanoma	Oncology	11
8	Ovarian Cancer	Oncology	10
9	Acute Leukemia Myeloid (AML)	Oncology	8
10	Non-Hodgkin Lymphoma (NHL)	Oncology	4
11	Gastric Cancer	Oncology	17
12	COVID-19	Infectious Disease	NEW INDICATION
13	Influenza Virus	Infectious Disease	42
14	Esophageal Cancer	Oncology	35
15	Myelodysplastic Syndrome (MDS)	Oncology	25
16	Pain	Neurology	18
17	Sarcoma	Oncology	29
18	Hyperlipidemia	Endocrinology	65
19	Alzheimer's Disease (AD)	Neurology	6
20	Dry Eye	Ophthalmology	57
21	Multiple Myeloma (MM)	Oncology	13
22	Glioblastoma Multiforme (GBM)	Oncology	23
23	Liver Cancer	Oncology	15
24	Parkinson's Disease (PD)	Neurology	12
25	Rheumatoid Arthritis (RA)	Immunology	16
26	Idiopathic Thrombocytopenic Purpura (ITP)	Immunology	296
27	Non-alcoholic Steatohepatitis (NASH)	Hepatology	21
28	Bladder Cancer	Oncology	19
29	Optic Neuritis (ON)	Ophthalmology	313
30	Myasthenia Gravis	Immunology	210
31	Psoriasis	Dermatology	20
32	Diabetes	Endocrinology	2
33	Age-related Macular Degeneration (AMD)	Ophthalmology	22
34	Multiple Sclerosis (MS)	Immunology	24
35	Hodgkin's Disease	Oncology	39
36	Asthma	Inflammatory	30
37	Renal Cancer	Oncology	28
38	Dermatitis	Dermatology	46
39	Campylobacter Enteritis	Infectious Disease	432
40	Ulcerative Colitis	Gastroenterology	27
41	HIV / AIDS	Infectious Disease	38
42	Cystic Fibrosis (CF)	Pulmonology	128
43	Retinitis Pigmentosa	Ophthalmology	183
44	Crohn's Disease	Gastroenterology	52
45	Chronic Lymphocytic Leukemia (CLL)	Oncology	54
46	Chronic Obstructive Pulmonary Disease (COPD)	Pulmonology	114
47	Acute Leukemia Lymphocytic (ALL)	Oncology	26
48	Glioma	Oncology	55
49	Heart Failure	Cardiovascular	92
50	Grave's Disease	Immunology	371

Top 100 Hot Indications

2021 RANK	INDICATION	THERAPEUTIC AREA	2020 RANK
51	Wounds	Dermatology	59
52	Pemphigus Vulgaris (PV)	Immunology	112
53	Pulmonary Fibrosis	Pulmonology	64
54	Neuropathy	Neurology	106
55	Myelofibrosis	Hematology	93
56	Hemophilia	Hematology	79
57	Amyotrophic Lateral Sclerosis (ALS)	Neurology	56
58	Epilepsy	Neurology	66
59	Hepatitis B Virus (HBV)	Hepatology	51
60	Cervical Cancer	Oncology	33
61	Inflammatory Bowel Disease (IBD)	Gastroenterology	36
62	Stroke	Neurology	75
63	Brain Cancer	Oncology	127
64	Depression	Psychiatry	61
65	Glaucoma	Ophthalmology	124
66	Graft-versus-host Disease (GvHD)	Transplant	110
67	Macular Edema	Ophthalmology	67
68	Addiction	Psychiatry	135
69	Anemia	Hematology	32
70	Schizophrenia	Psychiatry	70
71	Biliary Cancer	Oncology	34
72	Dermal Ulcers	Dermatology	73
73	Uterine Cancer	Oncology	37
74	Non-alcoholic Fatty Liver Disease (NAFLD)	Hepatology	58
75	Diabetic Retinopathy	Ophthalmology	82
76	Pulmonary Hypertension	Pulmonology	76
77	Gram-negative Bacterial Infection	Infectious Disease	134
78	Skin Cancer	Oncology	41
79	Liver Fibrosis	Hepatology	118
80	Diabetic Nephropathy	Nephrology	173
81	Chemotherapy-induced Side Effects	Oncology	203
82	Cirrhosis	Hepatology	157
83	Pompe's Disease	Endocrinology	98
84	Myocardial Infarction (MI)	Cardiovascular	101
85	Metastases	Oncology	178
86	Respiratory Syncytial Virus (RSV)	Infectious Disease	81
87	Acute Kidney Injury	Nephrology	88
88	Osteoporosis	Musculoskeletal	145
89	Respiratory Distress Syndrome (RDS)	Pulmonology	192
90	Hepatitis C Virus (HCV)	Hepatology	149
91	Primary Biliary Cirrhosis (PBC)	Hepatology	181
92	Kidney Transplant	Nephrology	48
93	Human Papillomavirus (HPV)	Urology & Women's Health	166
94	Scleroderma	Immunology	68
95	Duchenne Muscular Dystrophy	Musculoskeletal	86
96	Huntington's Disease (HD)	Neurology	117
97	Uveitis	Ophthalmology	174
98	Guillain-Barré Syndrome	Neurology	561
99	Osteoarthritis	Immunology	108
100	Ebola	Infectious Disease	119

Looking Forward

The past year was a unique year for clinical development. This year's first Hot MOAs give us a fresh perspective into upcoming developments and new trends to watch for across multiple therapeutic areas. With the evolving COVID-19 response still creating ripples throughout the pharma and biotech landscape, we're excited to see how these MOAs evolve and what new MOAs emerge next year.

Methodology

Our Hot Indications analysis framework considers the volume of ongoing scientific investigation, as well as the types of companies and levels of funding supporting these trials. Our analysis evaluated 42,957 drug programs ongoing in 2020, categorized them into 599 unique indications, and compared available data for these indications across three main criteria:

- 1. Pipeline Score:** The Pipeline Score measures the overall level of drug development activity for an indication. The score gives greater value to later-stage programs, higher volumes of programs overall, and indications with greater numbers of companies with programs.
- 2. R&D Funding:** Funding estimates the availability of financing to support the development of each drug program to its reasonable endpoint. For some programs, this endpoint will be FDA approval. In contrast, for others, it will be discontinuation in pre-clinicals or Phase I. The score measures availability of funds and willingness to invest based on two main inputs for each indication. First, the R&D Funding Score quantifies the historical track record of sponsor companies, based on the number of drugs each company has successfully developed. Second, the score measures initial public offering and venture capital investment fundraising activity in 2020 for each indication, with the expectation that the financing from such events will be major contributors in supporting ongoing R&D programs.
- 3. Academic Focus:** Academic Focus measures the overall publication activity for each indication, based on the absolute number and the one-year change in publications citing the indication for the evaluation period.

Hot Indications Ranking

For each of the 599 indications, we calculate the overall ranking score by a weighted average of Pipeline Score (50%), R&D Funding (40%) and Academic Focus (10%).

Therapeutic Areas

Each indication is categorized into one of 20 TAs, including an Other category. In general, indications are categorized based on the medical specialty most likely to treat patients with a disease or disorder. Systemic diseases, such as autoimmune disorders, or TAs representing a variety of medical specialties, such as Musculoskeletal, are grouped on a case-by-case basis. For example, Crohn's disease and ulcerative colitis are included in gastroenterology rather than immunology, whereas MS is included in immunology due to the variety of symptoms it presents. COVID-19 was a novel indication in 2020 and was included under infectious disease.

Hot MOA Methodology

To prioritize emerging MOAs, only MOAs being investigated in Phase I or Phase II studies were included. For each of the 2,335 MOAs in our data set, we calculated the number of unique indications and unique TAs associated with it. This analysis generated a ranked list of MOAs which were manually curated to identify those expanding into new indications and new TAs.



About Kx

For more than 40 years, Kx Advisors (operating as Kaiser Associates' healthcare practice) has provided strategy consulting services to help healthcare executives achieve critical business growth goals. With a highly collaborative approach they have developed pragmatic solutions for leading pharmaceutical, biotechnology, medical device, health IT, and digital health clients, with data-driven insights to give those clients the tools to compete and win across the healthcare industry.

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