

HOT INDICATIONS LIST 2017

Kaiser's 3rd annual analysis of global R&D investment has identified the top indications and therapeutic areas (TAs) for industry focus in 2016. This year, we're focusing on the changing funding landscape and the events underlying those changes. Here, we've highlighted the most dynamic TAs and the indications where investment has changed most since 2015, affecting rank in the Hot Indications List. As an example of the impact that funding events can have, \$474M in equity funding for Moderna Therapeutics affects its early-stage programs for a range of indications, including diabetic foot ulcers and zika virus, to name a few. To learn more about Kaiser's analysis methodology, see Page 6.



ONCOLOGY

Oncology attracts the greatest investment: in 2016, significant movement in melanoma, colorectal and pancreatic cancer was driven by investment in both innovation and biosimilars, e.g. Innovent Biologics' \$260M VC

2016 Rank	Indication	2015-16 Rank Change
6	Melanoma	↑ 23
7	Colorectal cancer	↑ 18
8	Pancreatic cancer	↑ 3
22	Head & neck cancers	↑ 27
27	Gastric cancer	↑ 108

HER2-targeted therapeutics have attracted investment, including \$62M VC funding for Zymeworks and \$100M for Hengrui Therapeutics



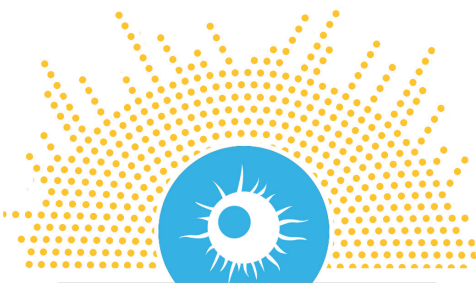
OPHTHALMOLOGY

Investment in specialty biotech boosts the TA: \$68M for Kala Pharmaceuticals (post-surgical eye pain, dry eye) and \$50M for Clearside Biomedical (retinal vein occlusion, macular edema)

49	Dry eye	↑ 112
68	Macular edema	↑ 80
88	Cataract	↑ 301
95	Retinal vein occlusion	↑ 142
194	Leber's hereditary optic neuropathy	↑ N/A

GenSight's €40M IPO to fund gene therapy development for LHON, retinitis pigmentosa, and AMD debuts this indication on the Hot Indications List

Orphan indication attracting interest from both established players, as Alexion seek label expansion for Soliris, and specialty biotech



IMMUNOLOGY

10	Rheumatoid arthritis (RA)	↑ 3
23	Multiple sclerosis (MS)	↑ 18
36	Lupus	↑ 18
48	Osteoarthritis	↑ 37
91	Myasthenia gravis	↑ 122

While GSK & Janssen's sirukumab struggle with FDA safety concerns, the competitive RA market shows no signs of slowing down, with recent approval of Sanofi & Regeneron's Kevzara



DERMATOLOGY

Continued big pharma innovation, biosimilars, and new players such as Kadmon's KD025 ROCK2 inhibitor (\$75M IPO), affect established markets psoriasis and RA

20	Psoriasis	↑ 6
47	Dermal ulcers	↑ 140
56	Dermatitis	↑ 7
125	Epidermolysis bullosa	↑ 358
192	Mastocytosis	↑ 343

Interest is high despite recent failure of Amicus' SD-101, as Amryt and Castle Creek gain funding for pivotal trials and Fibrocell begins clinical development of their gene therapy

TOP 10 MOVERS

77 Anaphylaxis
2015 Rank: 556
Rank Change: ↑479

19 Ulcerative colitis
2015 Rank: 385
Rank Change: ↑366

155 Intermittent claudication
2015 Rank: 514
Rank Change: ↑359

125 Epidermolysis bullosa
2015 Rank: 483
Rank Change: ↑358

65 Hyperphosphatemia
2015 Rank: 410
Rank Change: ↑345

192 Mastocytosis
2015 Rank: 535
Rank Change: ↑343

110 Short bowel syndrome
2015 Rank: 421
Rank Change: ↑311

88 Cataract
2015 Rank: 389
Rank Change: ↑301

163 Severe combined immunodeficiency
2015 Rank: 443
Rank Change: ↑280

193 Vasculitis
2015 Rank: 411
Rank Change: ↑218

2016 Ranking	Indication	Therapeutic Area	2015 Ranking	Change
1	Breast cancer	Oncology	2	↑ 1
2	Non-small cell lung cancer (NSCLC)	Oncology	3	↑ 1
3	Diabetes	Endocrinology	1	↓ -2
4	Non-Hodgkin's lymphoma (NHL)	Oncology	15	↑ 11
5	Prostate cancer	Oncology	10	↑ 5
6	Melanoma	Oncology	9	↑ 3
7	Colorectal cancer	Oncology	25	↑ 18
8	Pancreatic cancer	Oncology	11	↑ 3
9	Pain	Neurology	4	↓ -5
10	Rheumatoid arthritis (RA)	Immunology	13	↑ 3
11	Ovarian cancer	Oncology	8	↓ -3
12	Glioblastoma multiforme (GBM)	Oncology	508	↑ 496
13	Acute myelogenous leukemia (AML)	Oncology	7	↓ -6
14	Chronic lymphocytic leukemia (CLL)	Oncology	30	↑ 16
15	Liver cancer	Oncology	12	↓ -3
16	Multiple myeloma (MM)	Oncology	17	↑ 1
17	Asthma	Inflammatory	37	↑ 20
18	Alzheimer's disease (AD)	Neurology	5	↓ -13
19	Ulcerative colitis	Gastroenterology	385	↑ 366
20	Psoriasis	Dermatology	26	↑ 6
21	Renal cancer	Oncology	46	↑ 25
22	Head & neck cancers	Oncology	49	↑ 27
23	Multiple sclerosis (MS)	Immunology	41	↑ 18
24	Hyperlipidemia	Endocrinology	219	↑ 195
25	Non-alcoholic steatohepatitis (NASH)	Hepatology	27	↑ 2
26	Myelodysplastic syndrome (MDS)	Oncology	81	↑ 55
27	Gastric cancer	Oncology	135	↑ 108
28	Chronic obstructive pulmonary disease (COPD)	Pulmonology	24	↓ -4
29	Parkinson's disease (PD)	Neurology	21	↓ -8
30	Pulmonary fibrosis	Pulmonology	45	↑ 15
31	Lung cancer, general	Oncology	22	↓ -9
32	Influenza virus	Infectious Disease	35	↑ 3
33	Depression	Psychiatry	93	↑ 60
34	Wounds	Dermatology	143	↑ 109
35	Congestive heart failure (CHF)	Cardiovascular	199	↑ 164
36	Lupus	Immunology	54	↑ 18
37	HIV / AIDS	Infectious Disease	14	↓ -23
38	Acute lymphoblastic leukemia (ALL)	Oncology	163	↑ 125
39	Stroke	Neurology	78	↑ 39
40	Cystic fibrosis (CF)	Pulmonology	29	↓ -11
41	Age-related macular degeneration (AMD)	Ophthalmology	40	↓ -1
42	Crohn's disease	Gastroenterology	56	↑ 14
43	Small cell lung cancer	Oncology	73	↑ 30
44	Amyotrophic lateral sclerosis (ALS)	Neurology	33	↓ -11
45	Staphylococcus	Infectious Disease	125	↑ 80
46	Obesity	Endocrinology	58	↑ 12
47	Dermal ulcers	Dermatology	187	↑ 140
48	Osteoarthritis	Immunology	85	↑ 37
49	Dry eye	Ophthalmology	161	↑ 112
50	Rhinitis	ENT	137	↑ 87

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INNOVATIONS IN ONCOLOGY?

SEE HOT INDICATIONS DEEP DIVE: ONCOLOGY

<http://info.kaiserassociates.com/2017-hot-indications-deep-dive-oncology>

2016 Ranking	Indication	Therapeutic Area	2015 Ranking	Change
51	Neuropathy	Neurology	103	↑ 52
52	Pulmonary hypertension	Pulmonology	N/A	New in 2016
53	Glioma	Oncology	N/A	New in 2016
54	Hodgkin's disease	Oncology	108	↑ 54
55	Chemotherapy-induced side effects	Oncology	N/A	New in 2016
56	Dermatitis	Dermatology	63	↑ 7
57	Ataxia	Neurology	53	↓-4
58	Chronic kidney disease (CKD)	Nephrology	64	↑ 6
59	Anemia	Hematology	65	↑ 6
60	Peripheral vascular disease (PVD)	Cardiovascular	83	↑ 23
61	Anxiety	Psychiatry	168	↑ 107
62	Addiction	Psychiatry	98	↑ 36
63	Irritable bowel syndrome	Gastroenterology	156	↑ 93
64	Skin and skin structure infection (SSSI)	Dermatology	42	↓-22
65	Hyperphosphatemia	Hematology	410	↑ 345
66	Schizophrenia	Psychiatry	116	↑ 50
67	Inflammatory bowel disease (IBD)	Gastroenterology	16	↓-51
68	Macular edema	Ophthalmology	148	↑ 80
69	Endometriosis	Urology & Women's Health	222	↑ 153
70	Epilepsy	Neurology	57	↓-13
71	Esophageal cancer	Oncology	111	↑ 40
72	Age-related frailty	Musculoskeletal	N/A	New in 2016
73	Hepatitis C virus (HCV)	Hepatology	92	↑ 19
74	Bone repair	Musculoskeletal	76	↑ 2
75	Traumatic brain injury	Neurology	N/A	New in 2016
76	Graft-versus-host disease (GvHD)	Transplant	48	↓-28
77	Anaphylaxis	Inflammatory	556	↑ 479
78	Trypanosome	Infectious Disease	277	↑ 199
79	Glaucoma	Ophthalmology	158	↑ 79
80	Respiratory infection	Pulmonology	268	↑ 188
81	Neuroendocrine tumors	Oncology	71	↓-10
82	Bladder cancer	Oncology	59	↓-23
83	Sarcoma	Oncology	70	↓-13
84	Eye inflammation	Ophthalmology	460	↑ 376
85	Chronic myelogenous leukemia (CML)	Oncology	244	↑ 159
86	Uterine fibroids	Urology & Women's Health	177	↑ 91
87	Acne	Dermatology	31	↓-56
88	Cataract	Ophthalmology	389	↑ 301
89	Waldenström's macroglobulinemia	Oncology	N/A	New in 2016
90	Menopause	Endocrinology	175	↑ 85
91	Myasthenia gravis	Immunology	213	↑ 122
92	Hepatitis B virus (HBV)	Hepatology	126	↑ 34
93	Fallopian tube cancer	Oncology	N/A	New in 2016
94	Hypertension	Cardiovascular	23	↓-71
95	Retinal vein occlusion	Ophthalmology	237	↑ 142
96	Sepsis	Infectious Disease	182	↑ 86
97	Huntington's disease (HD)	Neurology	107	↑ 10
98	Paroxysmal nocturnal hemoglobinuria (PNH)	Hematology	139	↑ 41
99	Aspergillus	Infectious Disease	141	↑ 42
100	Tuberculosis	Infectious Disease	140	↑ 40



2016 Ranking	Indication	Therapeutic Area	2015 Ranking	Change
101	Nonalcoholic fatty liver disease (NAFLD)	Hepatology	N/A	New in 2016
102	Liver fibrosis	Hepatology	165	↑ 63
103	Gram-negative bacterial infection	Infectious Disease	112	↑ 9
104	Spinal cord injury (SCI)	Neurology	128	↑ 24
105	Myocardial infarction (MI)	Cardiovascular	82	↓-23
106	Cachexia	Endocrinology	124	↑ 18
107	Cushing's disease	Other	185	↑ 78
108	Vaccine adjuvancy	Immunology	N/A	New in 2016
109	Gram-positive bacterial infection	Infectious Disease	147	↑ 38
110	Short bowel syndrome	Gastroenterology	421	↑ 311
111	Neutropenia	Hematology	179	↑ 68
112	Down syndrome	Neurology	N/A	New in 2016
113	Drug toxicity	Other	N/A	New in 2016
114	Cervical cancer	Oncology	69	↓-45
115	Malaria	Infectious Disease	146	↑ 31
116	Osteoporosis	Musculoskeletal	34	↓-82
117	Organ transplant rejection	Transplant	274	↑ 157
118	Autism	Psychiatry	157	↑ 39
119	Diarrhea (infectious)	Gastroenterology	245	↑ 126
120	Hemophilia	Hematology	47	↓-73
121	T-cell leukemia	Oncology	N/A	New in 2016
122	Mesothelioma	Oncology	62	↓-60
123	Diabetic neuropathy	Neurology	227	↑ 104
124	Psoriatic arthritis	Immunology	214	↑ 90
125	Epidermolysis bullosa	Dermatology	483	↑ 358
126	Diabetic nephropathy	Nephrology	118	↓-8
127	Neuromyelitis optica (NMO)	Neurology	404	↑ 277
128	Polycystic ovarian syndrome	Urology & Women's Health	N/A	New in 2016
129	Duchenne muscular dystrophy	Musculoskeletal	N/A	New in 2016
130	Growth hormone deficiency	Endocrinology	113	↓-17
131	Brain cancer	Oncology	6	↓-125
132	Metastases	Oncology	189	↑ 57
133	Scleroderma	Immunology	122	↓-11
134	Diabetic retinopathy	Ophthalmology	224	↑ 90
135	Dementia	Neurology	129	↓-6
136	Myotonic dystrophy	Musculoskeletal	N/A	New in 2016
137	Rabies	Infectious Disease	101	↓-36
138	Smoking cessation	Pulmonology	N/A	New in 2016
139	Thrombocytopenia	Hematology	230	↑ 91
140	Coronary artery disease (CAD)	Cardiovascular	203	↑ 63
141	Sickle cell disease	Hematology	90	↓-51
142	Bacterial infection	Infectious Disease	28	↓-114
143	Acute leukemia	Oncology	N/A	New in 2016
144	Bone marrow transplant (BMT)	Transplant	270	↑ 126
145	Spinal muscular atrophy (SMA)	Neurology	95	↓-50
146	Kidney transplant	Nephrology	N/A	New in 2016
147	Human papillomavirus (HPV)	Urology & Women's Health	94	↓-53
148	Urinary tract infection (UTI)	Urology & Women's Health	84	↓-64
149	Urinary calculi / Kidney stones	Nephrology	N/A	New in 2016
150	Uveitis	Ophthalmology	132	↓-18

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151	Scars / wrinkles	Dermatology	212	↑ 61
152	Neuroblastoma	Oncology	N/A	New in 2016
153	Mitochondrial disease	Endocrinology	249	↑ 96
154	Hemolytic uremic syndrome	Hematology	181	↑ 27
155	Intermittent claudication	Cardiovascular	514	↑ 359
156	Bipolar disorder	Psychiatry	275	↑ 119
157	Angioplasty (PTCA)	Cardiovascular	525	↑ 368
158	Atherosclerosis	Cardiovascular	87	↓-71
159	Benign prostatic hyperplasia (BPH)	Urology & Women's Health	209	↑ 50
160	Hyperuricemia / gout	Endocrinology	149	↓-11
161	Preeclampsia	Urology & Women's Health	378	↑ 217
162	Clostridium	Infectious Disease	80	↓-82
163	Severe combined immunodeficiency (SCID)	Endocrinology	443	↑ 280
164	Community acquired infections	Infectious Disease	N/A	New in 2016
165	Fibrillation	Cardiovascular	155	↓-10
166	Adrenocortical carcinoma	Oncology	505	↑ 339
167	Erectile dysfunction (ED)	Urology & Women's Health	238	↑ 71
168	Anesthesia	Neurology	338	↑ 170
169	Radiotherapy-induced side effects	Oncology	N/A	New in 2016
170	Post-traumatic stress disorder	Psychiatry	204	↑ 34
171	Congenital adrenal hyperplasia	Endocrinology	N/A	New in 2016
172	Cytomegalovirus (CMV)	Infectious Disease	201	↑ 29
173	Onychomycosis	Infectious Disease	332	↑ 159
174	Niemann-Pick disease	Endocrinology	N/A	New in 2016
175	Polycystic kidney disease (PKD)	Nephrology	79	↓-96
176	Guillain-Barré syndrome	Neurology	N/A	New in 2016
177	Glomerulonephritis	Nephrology	340	↑ 163
178	Buerger's disease	Cardiovascular	550	↑ 372
179	Dental infections	Infectious Disease	N/A	New in 2016
180	Ankylosing spondylitis	Immunology	86	↓-94
181	Thyroid cancer	Oncology	215	↑ 34
182	Hidradenitis suppurativa	Dermatology	335	↑ 153
183	Cervical dysplasia	Urology & Women's Health	522	↑ 339
184	Herpes	Infectious Disease	N/A	New in 2016
185	Skin cancer	Oncology	198	↑ 13
186	Bone cancer	Oncology	235	↑ 49
187	Uterine cancer	Oncology	N/A	New in 2016
188	Hepatitis D virus (HDV)	Hepatology	183	↓-5
189	Dengue fever	Infectious Disease	97	↓-92
190	Allergic conjunctivitis	Ophthalmology	234	↑ 44
191	Incontinence	Urology & Women's Health	115	↓-76
192	Mastocytosis	Dermatology	535	↑ 343
193	Vasculitis	Inflammatory	411	↑ 218
194	Leber's hereditary optic neuropathy	Ophthalmology	N/A	New in 2016
195	Cough	PULmonology	166	↓-29
196	Alopecia	Dermatology	131	↓-65
197	Acute kidney injury	Nephrology	N/A	New in 2016
198	Biliary cancer	Oncology	88	↓-110
199	Itch	Dermatology	229	↑ 30
200	Attention deficit hyperactivity disorder (ADHD)	Psychiatry	50	↓-150



METHODOLOGY

Kaiser Associates' methodology is designed to assess investment intensity of drug development for each indication through a comprehensive and balanced analysis of the key drivers and metrics.

Our Hot Indications analysis framework considers the volume of ongoing scientific investigation, as well as the types of companies and level of funding supporting these trials. Kaiser's analysis evaluated 36,538 drug programs ongoing in 2016, categorized them into 626 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

R&D 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, while for others it will be discontinuation in preclinical 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 FundingScore 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 2016 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 626 indications, the overall ranking score is calculated 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 21 TAs, which include 20 major fields of medicine and an "Other" group. 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 Autoimmune, whereas Multiple Sclerosis is included in Autoimmune due to the variety of symptoms it presents.



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Founded in 1981, Kaiser Associates is an international strategy consulting firm that serves as a key advisor to the world’s leading companies. We provide our clients with the unique insight to drive critical decision-making and solve their most pressing problems.

Kaiser’s Global Healthcare Practice engages with executives at leading Life Sciences companies, including pharmaceutical, medical device, clinical diagnostics, consumer health, and health IT. We work with our clients to identify new growth markets, develop long-term portfolio strategies, and maximize commercial success.

The foundation of Kaiser’s service offering is its world-class “outside-in” methodology, which involves delivering critical facts and insights from the complex external environment to drive strategic decision-making. Kaiser possesses the unique ability to generate insights across physicians, thought leaders, patients, competitors, partners, regulators, suppliers, and payers. Kaiser uses its deep industry experience and analytical tools to synthesize this diverse set of insights and develop high-impact solutions.

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