

Appendix 1 (as supplied by the authors): Drugs, trials, outcomes and outcome classification

Drug		Indication	Outcome used for approval	Outcome classification
Generic name	Brand name			
Abatacept	Orencia	Rheumatoid arthritis	Disease activity score	Clinical
Abiraterone acetate	Zytiga	Prostate cancer	Overall survival	Clinical
Acamprosate calcium	Campral	Maintenance of abstinence from alcohol	Rate of complete abstinence	Clinical
Aclidinium	Tudorza Genuair	Chronic obstructive pulmonary disease	Forced expiratory volume in 1 second	Surrogate
Afatinib	Giotrif	Metastatic adenocarcinoma	Progression-free survival	Surrogate
Aflibercept	Eylea	Macular degeneration	Proportion of patients who maintained vision	Clinical
Alemtuzumab	Mabcampath	B-cell chronic lymphocytic leukemia	Complete and partial response rates	Surrogate
Alglucosidase alfa	Myozyme	Pompe's Disease	Proportion of patients alive and free of invasive ventilator support	Clinical
Aliskiren fumarate	Rasilez	Hypertension	Sitting blood pressure measurements, mean sitting systolic blood pressure, and mean sitting diastolic blood pressure	Surrogate
Alitretinoin	Toctino	Hand eczema	Physician Global Assessment	Clinical
Alogliptin	Nesina	Type 2 diabetes	HbA1c level	Surrogate
Ambrisentan	Volibris	Pulmonary arterial hypertension	6-minute walk distance	Surrogate
Anidulafungin	Eraxis	Invasive candidiasis/candidemia	Combined clinical and microbiological response	Clinical
Antihemophilic Factor (Recombinant BDD)	Eloctate	Hemophilia A (congenital factor VIII deficiency)	Annualized number of bleeding episodes or annualized bleeding rate	Clinical
Apixaban	Eliquis	Venous thromboembolic events	Composite of all venous thromboembolic events and all-cause death	Clinical
Aprepitant	Emend	Acute and delayed nausea and vomiting associated with moderately emetogenic cancer chemotherapy	No emetic episodes and no use of rescue therapy	Clinical
Aripiprazole	Abilify	Schizophrenia; bipolar I disorder	Positive and Negative Syndrome Scale total score; PANSS positive subscale score; PANSS negative subscale score; Clinical Global Impression scale Severity of Illness score; Young Mania Rating Scale	Clinical
AS03-adjuvanted H5N1 influenza vaccine	Arepanrix H5N1	Influenza	Ability to induce antibodies against viral hemagglutinin	Surrogate
Asenapine	Saphris	Schizophrenia, bipolar I disorder	Positive and Negative Syndrome Scale	Clinical

Axitinib	Inlyta	Metastatic renal cell carcinoma	Progression-free survival	Surrogate
Azacitidine	Vidaza	Myelodysplastic syndrome, acute myeloid leukemia	Overall survival	Clinical
Azelaic acid	Finacea	Rosacea	Improvement in primary characteristics of rosacea	Clinical
Azelastine and fluticasone	Dymista	Seasonal allergic rhinitis	Reflective total nasal symptom score	Clinical
Azilsartan	Edarbi	Hypertension	24-hour mean systolic blood pressure	Surrogate
Aztreonam	Cayston	Cystic fibrosis patients with chronic pulmonary Pseudomonas aeruginosa infections	Improvement in respiratory symptoms as measured by the Respiratory Symptoms scale of the Cystic Fibrosis Questionnaire	Clinical
Belimumab	Benlysta	Systemic lupus erythematosus	≥4-point reduction in the SELENA-SLEDAI score; no new British Isles Lupus Assessment Group (BILAG) A organ domain score or 2 new BILAG B organ domain scores; no worsening (<0.30-point increase) in Physician's Global Assessment (PGA) score	Clinical
Bendamustine	Treanda	Non-Hodgkin lymphoma, lymphocytic leukaemia	Overall response rate, progression-free survival	Surrogate
Besifloxacin	Besivance	Bacterial conjunctivitis	Clinical resolution and microbiological eradication	Clinical
Bevacizumab	Avastin	Metastatic colorectal cancer	Duration of survival	Clinical
Boceprevir	Victrelis	Hepatitis C	Sustained virologic response	Surrogate
Bortezomib	Velcade	Multiple myeloma	Overall response rate	Surrogate
Bosutinib	Bosulif	Chronic myelogenous leukemia	Complete or partial cytogenetic response	Surrogate
Brentuximab	Adcentris	Hodgkin lymphoma	Proportion of patients who achieved complete response or partial response	Surrogate
C1 Esterase Inhibitor, Human	Berinert	Hereditary angioedema	Time to onset of relief of symptoms of an abdominal or facial attack	Clinical
Cabazitaxel	Jevtana	Metastatic prostate cancer	Overall survival	Clinical
Canagliflozin	Invokana	Type 2 diabetes	HbA1c level	Surrogate
Canakinumab	Ilaris	Cryopyrin-Associated Periodic Syndromes	Proportion of patients with disease flare	Clinical
Catridecacog	Tretten	Congenital Factor XIII A-subunit deficiency	Rate of bleeding episodes that required treatment with a FXIII-containing product	Surrogate
Catumaxomab	Removab	Malignant ascites	Time to first need for therapeutic ascites puncture or death	Clinical
Ceftobiprole medocaril	Zeftera	Complicated skin and skin structure infections	Clinical cure rate	Clinical

Certolizumab pegol	Cimzia	Rheumatoid arthritis	American College of Rheumatology 20 response; Total Sharp Score	Clinical
Cetuximab	Erbitux	Metastatic colorectal cancer	Disease progression	Surrogate
Ciclesonide	Alvesco	Steroid-responsive bronchial asthma	Forced expiratory volume in one second and peak expiratory flow	Surrogate
Clevidipine	Cleviprex	Acute elevation of blood pressure in perioperative settings	Premature discontinuation of study drug	Surrogate
Clofarabine	Clolar	Acute lymphoblastic leukaemia	Overall remission rate	Surrogate
Colesevelam	Colesevelam	Frederickson Type IIa	Percent change in serum LDL-C concentration	Surrogate
Colesevelam hydrochloride	Lodalis	Frederickson Type IIa	Serum LDL-C concentration	Surrogate
Collagenase clostridium histolyticum	Xiaflex	Dupuytren's contracture	Reduction of contracture to 5° or less	Clinical
Crizotinib	Xalkori	Non-small cell lung cancer	Objective response rate	Surrogate
Dabigatran etexilate	Pradax	Venous thromboembolic events	Total venous thromboembolic events and all-cause mortality	Clinical
Dabrafenib	Tafinlar	Melanoma	Progression-free survival	Surrogate
Daptomycin	Cubicin	Complicated skin and skin structure infections; Staphylococcus aureus bloodstream infections	Clinical efficacy (improvement, cure)	Clinical
Darifenacin hydrobromide	Enablex	Overactive bladder	Number of incontinence episodes per week	Clinical
Darunavir	Prezista	HIV-1	Decrease in viral load of $\geq 1 \log_{10}$	Surrogate
Dasatinib	Sprycel	Myeloid leukemia	Hematologic or cytogenetic responses	Surrogate
Deferasirox	Exjade	Chronic iron overload	Reduction in liver iron concentration	Surrogate
Degarelix	Firmagon	Prostate cancer	Testosterone suppression to castrate levels	Surrogate
Denosumab	Prolia	Osteoporosis	New vertebral fractures	Clinical
Desvenlafaxine succinate	Pristiq	Major depressive disorder	17-item Hamilton Rating Scale for Depression	Clinical
Dexlansoprazole	Dexilant	Erosive esophagitis; symptomatic non-erosive gastroesophageal reflux disease	Percentage of subjects who had complete healing; percentage of patients with no daytime or nighttime heartburn	Clinical
Dexmedetomidine hydrochloride	Precedex	Intensive care unit sedation, conscious sedation	Observer's Assessment of Alertness/Sedation Scale <4	Clinical
Diclofenac diethylamine	Voltaren Emulgel	Pain associated with recent localized muscle or joint injuries	Pain intensity	Clinical
Dienogest	Visanne	Endometriosis	Pain intensity	Clinical

Difluprednate	Durezol	Post-operative inflammation following cataract surgery; endogenous anterior uveitis	Proportion of patients with anterior chamber (AC) cell count = 0; change from baseline in AC cell grade	Surrogate
Dimethyl fumarate	Tecfidera	Multiple sclerosis	Proportion of patients relapsed, annualized relapse rate	Clinical
Dolutegravir	Tivicay	HIV-1	HIV-1 RNA <50 copies/mL	Surrogate
Doripenem monohydrate	Doribax	Nosocomial pneumonia; complicated intra-abdominal infection; complicated urinary tract infections	Clinically evaluable patients at test of cure	Clinical
Doxycycline monohydrate	Efracea	Rosacea	Change in total inflammatory lesions in adults	Clinical
Dronedaron	Multaq	Atrial fibrillation	Time to first hospitalization or death	Clinical
Duloxetine hydrochloride	Cymbalta	Major depressive disorder; neuropathic pain associated with diabetic peripheral neuropathy	HAMD-17 total score; 24-hour average pain intensity/severity score	Clinical
Eculizumab	Soliris	Paroxysmal nocturnal haemoglobinuria	Hemoglobin stabilization and number of units of packed red blood cells transfused	Surrogate
Efalizumab	Raptiva	Plaque psoriasis	Psoriasis Area and Severity Index score	Clinical
Efinaconazole	Jublia	Onychomycosis	Complete cure rate	Clinical
Elosulfase alfa	Vimizim	Mucopolysaccharidosis IVA or Morquio A Syndrome	6 Minute Walk Test	Surrogate
Eltrombopag olamine	Revolade	Immune thrombocytopenic purpura	Platelet count $\geq 50 \times 10^9/L$	Surrogate
Elvitegravir, Cobicistat, Emtricitabine, and Tenofovir	Stribild	HIV-1	HIV-1 ribonucleic acid (RNA) <50 copies/mL	Surrogate
Emtricitabine	Emtriva	HIV-1	Proportion of patients with plasma HIV-1 RNA levels ≤ 50 copies/mL	Surrogate
Entecavir	Baraclude	Hepatitis B	Cell histology	Surrogate
Enzalutamide	Xtandi	Prostate cancer	Overall survival	Clinical
Eplerenone	Inspra	Myocardial infarction	Time to death from any cause and time to first occurrence of either cardiovascular mortality	Clinical
Eribulin mesylate	Halaven	Metastatic breast cancer	Overall survival	Clinical
Erlotinib	Tarceva	Non-small cell lung cancer	Overall survival	Clinical
Eslicarbazepine	Aptiom	Partial-onset seizures	Median percent reduction in seizure frequency, proportion of patients with a $\geq 50\%$ reduction in seizure frequency	Clinical
Etravirine	Intelence	HIV-1	Confirmed undetectable plasma viral load values	Surrogate
Everolimus	Afinitor	Metastatic renal cell carcinoma	Progression-free survival	Surrogate

Exetanide	Byetta	Type 2 diabetes	HbA1c level	Surrogate
Ezogabine	Potiga	Epilepsy	Seizure frequency	Clinical
Fampridine	Fampyra	Multiple sclerosis	Walking speed	Clinical
Febuxostat	Uloric	Gout	Serum uric acid levels of <6 mg/dL	Surrogate
Ferumoxytol	Feraheme	Iron deficiency anaemia with chronic kidney disease	Mean change in hemoglobin	Surrogate
Fesoterodine	Toviaz	Overactive bladder	Number of micturitions per 24 hours, number of urge urinary incontinence episodes	Clinical
Fidaxomicin	Dificid	Clostridium difficile infection	Improvement in diarrhoea or other symptoms such that further treatment was not needed	Clinical
Fingolimod	Gilenya	Multiple sclerosis	Annualized relapse rate	Clinical
Fluticasone furoate	Avamys	Seasonal allergic rhinitis	Nasal symptoms using the reflective total nasal symptom score	Clinical
Fluticasone furoate and vilanterol	Breo Ellipta	Airflow obstruction in patients with chronic obstructive pulmonary disease	Annual rate of moderate and severe exacerbations	Clinical
Galsulfase	Naglazyme	Mucopolysaccharidosis VI	Distance walked in the 12 minute walk test	Surrogate
Golimumab	Simponi	Rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis	American College of Rheumatology 20 response, Health Assessment Questionnaire score	Clinical
Guanfacine	Intuniv XR	Attention Deficit Hyperactivity Disorder	Attention Deficit Hyperactivity Disorder - Rating Scale-4th Edition	Clinical
Histrelin acetate	Vantas	Prostate cancer	Serum leutenizing hormone, prostate-specific antigen and prostatic acid phosphatase levels	Surrogate
Human Papillomavirus vaccine Types 16 and 18 (Recombinant, AS04 adjuvanted)	Cervarix	Cervical cancer	Protection against CIN2+ lesions	Surrogate
Human rotavirus, live, attenuated oral vaccine	Rotarix	Gastroenteritis caused by rotavirus	Protecting against severe rotavirus gastroenteritis episodes	Clinical
I 131 Tositumomab	Bexxar Therapy	Non-Hodgkin's lymphoma	General and complete response rate	Surrogate
Ibritumomab tiuxetan	Zevalin	Non-Hodgkin's lymphoma	Overall response rate	Surrogate
Icatibant	Firazyr	Hereditary angioedema	Time to onset of symptom relief	Clinical
Idebenone	Catena	Friedreich's Ataxia	Biochemical marker 8OH2'dG	Surrogate
Idursulfase	Elaprase	Hunter syndrome (Mucopolysaccharidosis II, MPS II)	Six-minute walk test; the percent-predicted forced vital capacity (FVC)	Surrogate
Indacaterol	Onbrez Breezhaler	Chronic obstructive pulmonary disease	24-hour post-dose trough FEV1	Surrogate

Infliximab	Remsima	Rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis, plaque psoriasis	American College of Rheumatology 20 response, Assessment of SpondyloArthritis International Society criteria,	Clinical
Influenza Vaccine (live, attenuated)	Flumist	Influenza	Efficacy against culture-confirmed influenza illness	Clinical
Ingenol	Picato	Actinic keratosis	Complete clearance rate	Clinical
Insulin Detemir	Levemir	Type 1 or 2 diabetes	HbA1c level	Surrogate
Insulin glulisine	Apidra	Type 1 or 2 diabetes	Change in glycosylated hemoglobin	Surrogate
Ipilimumab	Yervoy	Melanoma	Overall survival	Clinical
Japanese encephalitis vaccine (inactivated, adsorbed)	Ixiaro	Japanese encephalitis	Immune response	Surrogate
Lacosamide	Vimpat	Epilepsy	Seizure frequency	Clinical
Lanreotide	Somatuline Autogel	Acromegaly	Decrease in mean growth hormone of >50%	Surrogate
Lanthanum carbonate hydrate	Fosrenol	End stage renal disease	Reduction of serum phosphate	Surrogate
Lapatinib ditosylate	Tykerb	Metastatic breast cancer	Time to progression	Surrogate
Lenalidomide	Revlimid	Myelodysplastic syndromes	Need for red blood cell transfusions	Surrogate
Linacotide	Constella	Irritable bowel syndrome	30% reduction from baseline in mean abdominal pain; at least 3 complete spontaneous bowel movements and an increase of at least 1 complete spontaneous bowel movement from baseline	Clinical
Linagliptin	Trajenta	Type 2 diabetes	HbA1c level	Surrogate
Liraglutide	Victoza	Type 2 diabetes	HbA1c level	Surrogate
Lisdexamfetamine dimesylate	Vyanse	Attention Deficit Hyperactivity Disorder	ADHD Rating Scale	Clinical
Lomitapide	Juxtapid	Homozygous familial hypercholesterolemia	Low-density lipoprotein cholesterol	Surrogate
Loteprednol etabonate	Alrex	Seasonal allergic conjunctivitis	Bulbar conjunctival injection and ocular itching	Clinical
Lumiracoxib	Prexige	Osteoarthritis of the knee	Pain intensity, patient's global assessment of disease activity, and the total score of the Western Ontario and McMaster Universities questionnaire	Clinical
Lurasidone	Latuda	Schizophrenia	Brief Psychiatric Rating Scale total score and mean change in the Positive and Negative Syndrome Scale total score	Clinical

Lutropin alpha	Luveris	Stimulation of follicular development	At least one follicle \geq 17 mm, pre-ovulatory E2 serum level \geq 109 pg/ml (400 pmol/L), and mid-luteal phase P4 level \geq 7.9 ng/mL (25nmol/L)	Surrogate
Macitentan	Opsumit	Pulmonary arterial hypertension	Time to first occurrence of a morbidity or mortality event	Clinical
Maraviroc	Celsentri	HIV-1	Mean change from baseline HIV-1 RNA	Surrogate
Meningococcal (Groups A, C, W-135 and Y) Oligosaccharide CRM197 Conjugate Vaccine	Menveo	Neisseria meningitidis	Immunogenicity response	Surrogate
Meningococcal polysaccharide groups A, C, W-135 and Y conjugate vaccine	Nimenrix	Neisseria (N.) meningitidis	rSBA-MenC titre	Surrogate
Methoxy polyethylene glycol epoetin beta solution	Mircera	Anemia associated with chronic kidney disease	Proportion of patients who experienced a hemoglobin response	Surrogate
Methyl aminolevulinate hydrochloride	Metvix	Actinic keratosis, basal cell carcinoma	Complete response rate	Clinical
Methylnaltrexone bromide	Relistor	Opioid-induced constipation	Rescue-free laxation	Clinical
Micafungin sodium	Mycamine	Esophageal candidiasis	Clinical and endoscopic cure	Clinical
Mirabegron	Myrbetriq	Overactive bladder	Mean number of incontinence episodes per 24 hours and mean number of micturitions per 24 hours	Clinical
Natalizumab	Tysabri	Multiple sclerosis	Expanded Disability Status Scale progression, annualized relapse rate	Clinical
Nebivolol	Bystolic	Mild to moderate essential hypertension	Change from baseline in trough sitting diastolic blood pressure	Surrogate
Nelarabine	Atriance	T-cell acute lymphoblastic leukemia and T-cell lymphoblastic lymphoma	Complete and partial response rates	Surrogate
Nepafenac	Nevanac	Pain and inflammation associated with cataract surgery	Absence of inflammation	Clinical
Nesiritide	Natrecor	Acute decompensated heart failure	Pulmonary capillary wedge pressure and dyspnea	Surrogate
Nilotinib hydrochloride monohydrate	Tasigna	Myeloid leukemia	Hematologic response	Surrogate
Nitric oxide	Inomax	Hypoxic respiratory failure	Incidence of death and/or receipt of extracorporeal membrane oxygenation	Clinical
Ocriplasmin	Jetrea	Vitreomacular adhesion	Vitreomacular adhesion resolution	Surrogate
Ofatumumab	Arzerra	Chronic lymphocytic leukaemia	Complete remission or partial remission	Surrogate
Olmesartan medoxomil	Olmetec	Hypertension	Trough sitting diastolic blood pressure	Surrogate

Olodaterol	Striverdi respimat	Chronic obstructive pulmonary disease	FEV1 area under the curve	Surrogate
Oxaliplatin	Eloxatin	Metastatic colorectal cancer	Median time to tumour progression	Surrogate
Palifermin	Kepivance	Oral mucositis	Duration of WHO grade 3-4 oral mucositis	Clinical
Paliperidone	Invega	Schizophrenia	Positive and Negative Syndrome Scale	Clinical
Paliperidone palmitate	Invega Sustenna	Schizophrenia	Positive and Negative Syndrome Scale	Clinical
Palonosetron	Aloxi	Acute and delayed nausea and vomiting associated with moderately emetogenic cancer chemotherapy	No emetic episode and no rescue medication within 24 hours following chemotherapy	Clinical
Panitumumab	Vectibix	Colorectal cancer	Progression free survival	Surrogate
Pantoprazole magnesium	Pantoloc M	Gastric and duodenal ulcers, reflux esophagitis, symptomatic gastro-esophageal reflux disease, and Helicobacter pylori associated duodenal ulcers	Healing of mucosal lesions	Clinical
Paricalcitol	Zemplar	Secondary hyperparathyroidism	Level of parathyroid hormone, values of serum Ca, PO ₄ , and CaXPO ₄ .product	Surrogate
Pasireotide	Signifor	Cushing's disease	Normalization of mean 24-hour urinary-free cortisol level	Surrogate
Pazopanib hydrochloride	Votrient	Renal cell carcinoma	Progression-free survival	Surrogate
Pegaptanib sodium injection	Macugen	Subfoveal choroidal neovascularization	Losing < 15 letters of best-corrected visual acuity	Clinical
Pegvisomant	Somavert	Acromegaly	Insulin-like growth factor-I levels	Surrogate
Perampanel	Fycompa	Epilepsy	Percent change in seizure frequency	Clinical
Pertuzumab	Perjeta	Metastatic breast cancer	Progression-free survival	Surrogate
Pirfenidone	Esbriet	Idiopathic pulmonary fibrosis	Predicted Forced Vital Capacity	Surrogate
Plerixafor	Mozobil	Non-Hodgkin's lymphoma, multiple myeloma	Target number of $\geq 5 \times 10^6$ CD34+ cells/kg	Surrogate
Pneumococcal 13-valent Conjugate Vaccine	Prevnar 13	Streptococcus pneumoniae	Serum anti-capsular polysaccharide immunoglobulin G antibody concentration	Surrogate
Pneumococcal conjugate vaccine	Synflorix	Streptococcus pneumoniae	Immune response	Surrogate
Pomalidomide	Pomalyst	Multiple myeloma	Progression-free survival	Surrogate
Posaconazole	Posanol	Aspergillus and Candida infections	Proven or probable invasive fungal infection	Clinical
Prasugrel hydrochloride	Effient	Atherothrombotic events in patients with acute coronary syndrome	Composite of cardiovascular death, nonfatal myocardial infarction, or nonfatal stroke	Clinical
Pregabalin	Lyrica	Neuropathic pain	End-point mean pain score	Clinical

Prucalopride	Resotran	Chronic idiopathic constipation	≥3 spontaneous complete bowel movements per week	Clinical
Quadrivalent Human Papillomavirus [Types 6, 11, 16, 18] (recombinant, adsorbed)	Gardasil	Cervical cancer, vulvar and vaginal cancer, genital warts, cervical adenocarcinoma in situ, cervical intraepithelial neoplasia grades 1, 2 and 3, vulvar intraepithelial neoplasia grades 2 and 3, and vaginal intraepithelial neoplasia	Reduction in genital warts	Clinical
Radium Ra 223 dichloride	Xofigo	Prostate cancer	Overall survival	Clinical
Raltegravir	Isentress	HIV-1	HIV RNA levels <400 copies/mL	Surrogate
Ranibizumab	Lucentis	Macular degeneration	Maintenance of vision	Clinical
Rasagiline mesylate	Azilect	Parkinson's disease	Unified Parkins's Disease Rating Scale	Clinical
Recombinant human coagulation factor IX-FC fusion protein	Alprolix	Congenital factor IX deficiency	Annualized bleeding rate per subject	Clinical
Recombinant Neisseria meningitidis serogroup B protein antigen, NHBA (Neisseria Heparin Binding Antigen) as a fusion protein, fHbp (factor H binding protein) as a fusion protein; and PorA P1.4 as the main antigen of Outer Membrane Vesicles (OMV) derived from Neisseria meningitidis serogroup B, strain NZ 98/254	Bexsero	Neisseria meningitidis serogroup B strains	Serum bactericidal assay using human complement (hSBA)	Surrogate
Regorafenib	Stivarga	Metastatic colorectal cancer	Overall survival	Clinical
Remestemcel-L	Prochymal	Graft versus Host Disease	Complete or partial cytogenetic response	Surrogate
Retapamulin	Altargo	Uncomplicated bacterial skin and skin structure infections due to Staphylococcus aureus (methicillin-susceptible isolates only) or Streptococcus pyogenes	Microbiological success rates	Clinical
Rifaximin	Zaxine	Hepatic encephalopathy	Time to first breakthrough overt hepatic encephalopathy episode	Clinical
Rilpivirine hydrochloride	Edurant	HIV-1	Proportion of patients with plasma viral load <50 copies/mL	Surrogate
Riociguat	Adempas	Chronic thromboembolic pulmonary hypertension	Distance covered in a 6-minute walking test	Surrogate test
Rivaroxaban	Xarelto	Venous thromboembolic events	Deep vein thrombosis, non-fatal pulmonary embolism, and death from all causes	Clinical

Rivastigmine	Exelon Patch	Dementia of the Alzheimer's type	Cognitive subscale of the Alzheimer's Disease Assessment Scale, and the Alzheimer's Disease Cooperative Study - Clinical Global Impression of Change	Clinical
Roflumilast	Daxas	Chronic obstructive pulmonary disease	Lung function; rate of moderate or severe exacerbations	Clinical
Romidepsin	Istodax	Peripheral T-cell lymphoma	Complete response/unconfirmed complete response	Surrogate
Romiplostim	Nplate	Chronic immune thrombocytopenic purpura	Durable platelet response	Surrogate
Rotavirus vaccine, live, pentavalent	Rotateq	Rotavirus gastroenteritis	Efficacy against all severities of rotavirus gastroenteritis	Clinical
Rotigotine	Neupro	Parkinson's disease	Unified Parkinson's Disease Rating Scale	Clinical
Rufinamide	Banzel	Seizures associated with Lennox-Gastaut syndrome (LGS)	Median percent change in total seizure frequency per 28 days; median percent change in tonic-atonic seizure frequency (drop attacks) per 28 days; seizure severity from the Parent/Guardian Global Evaluation of the patient's condition	Clinical
Ruxolitinib	Jakavi	Splenomegaly with primary myelofibrosis	≥35% reduction in spleen volume	Surrogate
Sapropterin dihydrochloride	Kuvan	Hyperphenylalaninemia	Blood Phe level	Surrogate
Saxagliptin hydrochloride	Onglyza	Type 2 diabetes	HbA1c level	Surrogate
Sildenafil	Rapaflo	Benign prostatic hyperplasia	International Prostate Symptom Score	Clinical
Simeprevir	Galexos	Hepatitis C	Sustained virologic response	Surrogate
Sitagliptin phosphate monohydrate	Januvia	Type 2 diabetes	HbA1c level	Surrogate
Sitaxsentan sodium	Thelin	Pulmonary arterial hypertension	Peak oxygen uptake during cycle ergometry	Surrogate
Sodium oxybate	Xyrem	Cataplexy	Total number of cataplexy attacks	Clinical
Sofosbuvir	Sovaldi	Hepatitis C	Sustained virologic response	Surrogate
Solifenacin succinate	Vesicare	Overactive bladder	Number of micturitions per 24 hours	Clinical
Sorafenib	Nexavar	Renal cell carcinoma	Progression-free survival	Surrogate
Stiripentol	Diacomit	Severe myoclonic epilepsy in infancy	≥50% decrease in the frequency of generalized clonic or tonic-clonic seizures	Clinical
Sunitinib malate	Sutent	Gastrointestinal stromal tumour	Time to tumour progression	Surrogate
Tafloprost	Saflutan	Elevated intraocular pressure	Mean diurnal intraocular pressure	Surrogate
Taliglucerase alfa	Elelyso	Type 1 Gaucher disease	Reduction in mean spleen volume	Surrogate
Tapentadol hydrochloride	Nucynta CR	Pain	Pain intensity	Clinical
Telaprevir	Incivek	Hepatitis C	Sustained virologic response	Surrogate

Telavancin hydrochloride	Vibativ	Complicated skin and skin structure infections	Clinical cure rate	Clinical
Telbivudine	Sebivo	Hepatitis B	HBV DNA suppression to <5 log ₁₀ copies per mL combined with either HBe antigen loss or ALT normalization	Surrogate
Temsirolimus	Torisel	Renal cell carcinoma	Overall survival	Clinical
Teriflunomide	Aubagio	Relapsing remitting multiple sclerosis	Number of confirmed relapses per patient-year	Clinical
Tesamorelin	Egrifta	Excess visceral adipose tissue in adults with HIV	Reduction of visceral adipose tissue	Surrogate
Thrombin alfa (Recombinant)	Recothrom	Haemostasis	Incidence of haemostasis	Surrogate
Ticagrelor	Brilinta	Atherothrombotic events in patients with acute coronary syndromes	Time to first event of cardiovascular death, myocardial infarction, and stroke	Clinical
Tigecycline	Tygasil	Complicated skin and skin structure infections, complicated intra-abdominal infections	Clinical cure rate	Clinical
Tipranavir	Aptivus	HIV-1	Proportion of patients with a treatment response $\geq 1 \log_{10}$ reduction in viral load	Surrogate
Tocilizumab	Actemra	Rheumatoid arthritis	Proportion of patients who achieved an American College of Rheumatology 20 response	Clinical
Tofacitinib	Xeljanz	Rheumatoid arthritis	American College of Rheumatology 20 response	Clinical
Tolvaptan	Samsca	Hyponatraemia	24-hour area under the curve in serum sodium	Surrogate
Trabectedin	Yondelis	Ovarian cancer	Progression-free survival	Surrogate
Tramadol hydrochloride	Tramacet	Pain	Total pain relief, sum of pain intensity difference, sum of total pain relief and sum of pain intensity differences	Clinical
Trametinib	Mekinist	Melanoma	Progression-free survival	Surrogate
Trastuzumab	Kadcyla	Metastatic breast cancer	Progression-free survival and overall survival	Clinical
Trospium chloride	Trosec	Overactive bladder	Number of toilet voids per 24 hours, urge incontinence episodes per 24 hours	Clinical
Ulipristal	Fibristal	Uterine fibroids	Percentage of women with a reduction in uterine bleeding	Clinical
Umeclidinium	Anoro Ellipta	Airflow obstruction in patients with chronic obstructive pulmonary disease	Trough forced expiratory volume in 1 second	Surrogate
Ustekinumab	Stelara	Plaque psoriasis	Psoriasis Area and Severity Index score	Clinical

Vandetanib	Caprelsa	Medullary thyroid cancer	Progression-free survival	Surrogate
Varenicline tartrate	Champix	Smoking-cessation	Abstinence responder rate	Clinical
Velaglucerase alfa	Vpriv	Type 1 Gaucher disease	Haemoglobin concentration	Surrogate
Vemurafenib	Zelboraf	Melanoma	Overall survival and progression-free survival	Clinical
Vismodegib	Erivedge	Metastatic basal cell carcinoma	Complete or partial response	Surrogate
Vorinostat	Zolinza	Cutaneous T-cell lymphoma	Modified Severity Weighted Assessment Tool	Clinical
Vortioxetine	Trintellix	Major depressive disorder	MADRS or HAM-D24 total score	Clinical
Ziprasidone hydrochloride monohydrate	Zeldox	Schizophrenia	Brief Psychiatric Rating Scale and Positive and Negative Syndrome Scale	Clinical
Zucapsaicin	Civanex	Severe pain in adults with osteoarthritis of the knee	Osteoarthritis pain of the knee and physical function, assessed using the Western Ontario and McMaster Universities Arthritis Index, and the Subject's Global Evaluation	Clinical