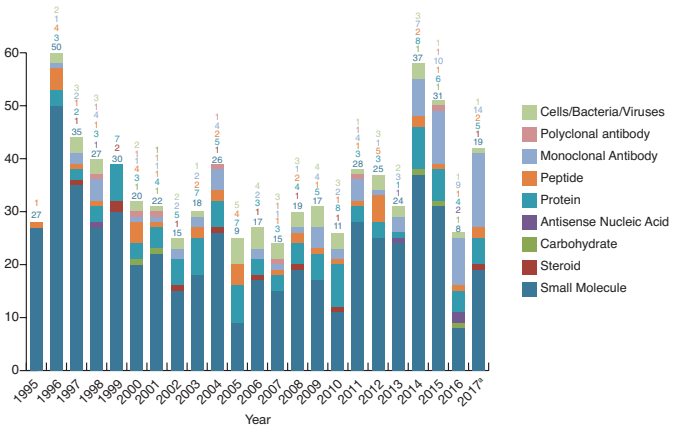
Drug pipeline 3Q17

Laura DeFrancesco

Drug approvals are way up through three quarters, including a number of first-in-class approvals. The first drug targeting a metabolic enzyme mutated in cancer (Celgene's mitochondrial isocitrate dehydrogenase) was approved. The approval of Novartis' highly anticipated CAR-T cancer therapy Kymriah for acute myelogenous leukemia was followed two months later by another: Gilead/Kite's Yescarta for diffuse large B-cell lymphomas. Several gene therapies attained breakthrough drug designation; the first AAV gene therapy approval for a retinal disorder looks likely before year's end.

Historic US regulatory approvals by lead indication



[a]2017 partial year ending September 30. Source: BioMedTracker a service of Sagient Research (http://www.biomedtracker.com)

Notable regulatory approvals (3Q17)

Drug/company	Indication	Drug information
Aliqopa (copanlisib)/ Bayer	Indolent non-Hodgkin lymphoma	9/14/2017 FDA granted accelerated approval for this small-molecule PI3K/AKT pathway inhibitor
Tremfya (guselkumab)/ J&J	Psoriasis	7/13/2017 FDA approved this fully human monoclonal antibody targeting only the p19 subunit of IL-23
Nerlynx (neratinib maleate)/Puma	Breast cancer	7/17/2017 FDA approved this small-molecule HER-2 inhibitor for women previously treated with Herceptin (trastuzumab) to prevent recurrence
Idhifa (enasidenib)/ Celgene	Acute myelogenous leukemia	8/1/2017 FDA approves this small-molecule first-in-class inhibitor of mitochondrial isocitrate dehydrogenase
Besponsa (inotuzumab ozogamicin)/Pfizer	Acute lymphocytic leukemia	8/17/2017 FDA approves this humanized IgG4 monoclonal antibody against CD22 conjugated with N-acetyl gamma calicheamicin
Mylotarg (gemtuzumab ozogamicin)/Pfizer	Acute myelogenous leukemia	9/1/2017 FDA approves this second try at humanized monoclonal IgG4 antibody against CD33 conjugated with calicheamicin
Kymriah (tisagenlecleucel-t)/Novartis	Acute lymphocytic leukemia	8/30/2017 FDA approved first-in-class CAR-T cells against CD-19 bearing cells
Verzenio (abemaciclib)/ Eli Lilly	Breast cancer	9/28/2017 FDA approves this small-molecule cyclin-dependent kinase (CDK)4 and CDK6 inhibitor
Breakthrough drug designation		
Venclexta (venetoclax)/ AbbVie	Acute myelogenous leukemia	Small-molecule Bcl-2 selective inhibitor
Imfinzi (durvalumab)/ AstraZeneca	Non-small cell lung cancer	Human IgG1κ monoclonal antibody directed against B7-H1 T-cell activator
Mogamulizumab/Kyowa Hakko Kirin	Cutaneous T-cell lymphoma	Humanized monoclonal antibody for CCR4 chemokine receptors selectively expressed in T-helper type 2 (Th2) cells
DS-8201/Daiichi Sankyo	Breast cancer	Humanized anti-HER2 monoclonal antibody conjugated to exatecan derivative
EB-101/Abeona Therapeutics	Epidermolysis bullosa	Autologous ex vivo gene therapy in which the COL7A1 gene in inserted into skin cells
ABO-101/Abeona Therapeutics	Mucopolysaccharidosis IIIB	Gene therapy with recombinant AAV9 expressing human alpha-N-acetylglucosaminidase
Cemiplimab/Regeneron	Skin cancer: squamous cell carcinoma	Fully human monoclonal antibody against PD1

J&J, Johnson and Johnson; FDA, US Food and Drug Administration; CAR-T, chimeric antigen receptor T cell; AAV, adeno-associated virus. Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com)

Notable regulatory setbacks (3Q17)

Drug/company	Indication	Drug information
Evenity (romosozumab)/ Amgen	Osteoporosis/osteopenia	7/16/2017 FDA issued a complete response letter for this humanized mAb against sclerostin, requesting data from other clinical trials
Suptavumab/Regeneron	Respiratory syncytial virus	8/14/2017 Company suspended phase 3 trial in preterm infants of fully human mAb against the fusion protein of RSV due to lack of efficacy
Sirukumab/J&J	Rheumatoid arthritis	9/22/2017 FDA issued a complete response letter for this humanized mAb against IL-6 due to safety concerns and a negative advisory opinion
Intepirdine/Axovant	Alzheimer's disease	9/26/2017 Company suspended phase 3 trial of small-molecule selective 5-HT6 receptor antagonist due to lack of improvement in cognition or in daily living activity
Translarna/PTC Therapeutics	Duchenne's muscular dystrophy	11/02/2017 FDA sent a complete response letter for this small-molecule exon-skipping drug

FDA, US Food and Drug Administration; mAb, monoclonal antibody; J&J, Johnson and Johnson. Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com)

Notable clinical trial results (3Q17)

Drug/company	Indication	Summary
Emicizumab/Roche	Hemophilia A	7/10/2017 In phase 3 randomized study, bispecific antibody against factor IXa and factor X reduced bleeding episodes by 87% compared with blocking agents. (*N. Engl. J. Med.* **377**, 809–818, 2017)
Translarna/PTC Therapeutics	Duchenne's muscular dystrophy	7/17/2017 In phase 3 randomized placebo controlled trial, this small-molecule exon-skipping drug improved six-minute walk distance in a subgroup compared to placebo. (*Lancet* **390**, 1489–1498, 2017)
Luxturna (AAV2.hRPE65v2)/Spark Therapeutics	Leber's congenital amaurosis	7/13/2017 In open label randomized phase 3 trial of gene therapy of human retinal pigment epithelium 65-kD protein, vision, light sensitivity and visual field was improved at one year. (*Lancet* **390**, 849–860, 2017)
Anacetrapib/Merck	Dyslipidemia/ hypercholesterolemia	8/29/2017 In phase 3 randomized, placebo-controlled trial of this cholesteryl ester transfer protein inhibitor, the mean level of non-HDL cholesterol was reduced by 18% and HDL cholesterol level increased by 104%, but major coronary events were only reduced by 9%. (*N. Engl. J. Med.* **377**, 217–1227, 2017)

Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com)

Notable upcoming regulatory catalysts (4Q17)

Drug/company	Indication	Summary
Ozenoxacin/Medimetriks	Skin and skin-structure infections	11/30/2017 FDA PDUFA date for this novel non-fluorinated quinolone antibacterial agent
LR769/LFB Group	Hemophilia A and B	11/22/2017 FDA PDUFA date for recombinant FVIIa, produced in the milk of transgenic rabbits to control bleeding in patients with inhibitors to coagulation factors
Andexxa (andexanet alfa)/Portola	Drug toxicity	11/30/2017 EMA CHMP decision on universal Factor Xa inhibitor antidote to address uncontrollable bleeding
Luxturna/Spark Therapeutics	Leber's congenital amaurosis	1/12/2018 FDA PDUFA date for this gene therapy of AAV-delivered human retinal pigment epithelium 65-kD protein
Macrilen (growth hormone secretagogue)/ Aeterna	Short stature	12/29/2017 FDA 2nd PDUFA date ghrelin receptor targeting growth hormone secretagogue
Benralizumab/ AstraZeneca	Asthma	12/31/2017 FDA PDUFA date for this humanized monoclonal antibody against IL-5Rα
Ibalizumab/ Theratechnologies	HIV/AIDS	1/3/2018 FDA PDUFA date for this humanized monoclonal antibody against CD4, which inhibits entry of HIV into CD4 cells
Lutathera (lutetium Lu 177 dotatate)/Advanced Accelerator Applications	Neuroendocrine tumors	1/26/2018 FDA PDUFA data for this radiolabeled somatostatin
VX-661 (tezacaftor)/ Vertex	Cystic fibrosis	2/28/2018 FDA PDUFA date for this small-molecule CFTR corrector
Emicizumab/Roche	Hemophilia A	2/23/2018 FDA PDUFA date for this bispecific IgG antibody against factor IXa and factor X
Tildrakizumab/Sun Pharmaceuticals	Psoriasis	3/1/2018 FDA PDUFDA date for this fully human monoclonal antibody IgG1κ CD23 mAb

FDA, US Food and Drug Administration; mAb, monoclonal antibody; EMA, European Medicines Agency; CHMP, The Committee for Medicinal Products for Human Use; PDUFA, Prescription Drug User Fee Act. Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com)

Laura DeFrancesco is Senior Editor at Nature Biotechnology.