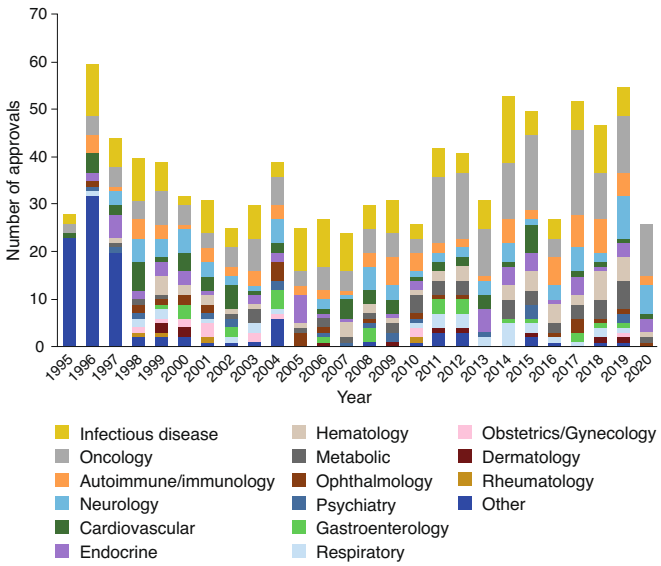
DATA PAGE

Drug pipeline 2Q20

The US Food and Drug Administration (FDA) picked up the pace of drug registrations after a sluggish first quarter. Many were accelerated approvals for cancer indications, including two small molecules — Incyte's Pemazyre and Novartis's Tabrecta — approved with companion diagnostics from Foundation Medicine. The agency halted two adeno-associated virus (AAV) gene therapy trials, from Audentes and Sarepta, for safety reasons; next quarter, the European Medicines Agency (EMA) will consider AAV gene therapies from Biomarin and PTC Therapeutics for hemophilia and aromatic L-amino acid decarboxylase (AADC) deficiency, respectively. A small interfering RNA (siRNA) oligonucleotide therapy for hypercholesterolemia from Novartis (The Medicines Company) also will be under evaluation. Several companies reported clinical results from drugs repurposed for COVID-19, with Gilead and Kiniksa reporting preliminary data showing improvements in duration of illness and certain outcomes for Veklury (remdesivir) and mavrilimumab, respectively.

Historic US regulatory approvals by drug class

Despite the pandemic, the rate of drug approval looks remarkably similar to previous years'.



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

Notable clinical trial results (1Q20)

Drug/company	Indication	Drug information
Enspryng (satralizumab)/ Roche	Neuromyelitis optica (Devic's syndrome)	4/24/2020 A double-blind, placebo-controlled trial of this humanized IgG2 mAb against IL-6 receptor reduced the risk of relapse by 55% (*Lancet Neurol.* **19**, 402–412, 2020)
Veklury (remdesivir)/ Gilead	COVID-19	5/22/2020 A double-blind, randomized, placebo-controlled trial of this small-molecule nucleotide analog viral RNA-dependent RNA polymerase inhibitor reduced recovery to 11 days, as compared with 15 days for placebo (*New Engl. J. Med.* https://doi.org/10.1056/NEJMoa2007764, 2020)
Mavrilimumab/ Kiniksa	COVID-19	6/16/2020 In an open-label clinical trial with this fully human IgG4 mAb against granulocyte macrophage colony stimulating factor receptor alpha in patients with severe disease, none of 13 patients treated with drug died while 7 (27%) in the control group died. (*Lancet Rheumatol.* https://doi.org/10.1016/S2665-9913(20)30170-3, 2020)

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

Notable drug approvals (2Q20)

Drug/company	Indication	Drug information
Pemazyre (pemigatinib)/ Incyte	Biliary tract cancer	4/17/2020 FDA grants accelerated approval for this small-molecule inhibitor of fibroblast growth factor receptor (FGFR)-1/2/3 tyrosine kinase for use in patients with *FGFR2* gene fusions along with a FoundationOne next-generation sequencing panel companion diagnostic
Trodelvy (sacituzumab govitecan)/ Immunomedics	Breast cancer	4/22/2020 FDA grants accelerated approval for this humanized IgG1κ monoclonal antibody (mAb) conjugated with 7 or 8 molecules of SN-38, a topoisomerase inhibitor, using hydrolysable linker CL2A
Tabrecta (capmatinib)/ Novartis	Non-small-cell lung cancer	1/21/2020 FDA grants accelerated approval for this small-molecule inhibitor of hepatocyte growth factor receptor (c-Met, HGFR) tyrosine kinase in patients with tumors harboring mutation leading to mesenchymal-epithelial transition (*MET*) exon 14 skipping as detected by next-generation sequencing panel companion diagnostic (FoundationOne)
Koselugo (selumetinib)/ AstraZeneca	Neurofibromatosis	4/10/2020 FDA approves this small-molecule selective, non-ATP-competitive inhibitor of MEK (MAP-ERK kinase)-1/2 for neurofibromatosis
Retevmo (selpercatinib)/ Eli Lilly	Non-small-cell lung cancer, thyroid cancer	5/8/2020 FDA grants accelerated approval for this small-molecule inhibitor of RET (rearranged during transfection) in patients with RET fusion mutations

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

Upcoming catalysts (3Q20)

Drug/company	Indication	Drug information
REGN-EB3 (EBOV glycoprotein)/ Regeneron	Ebola virus infection	10/23/2020 FDA PDUFA date for cocktail of three human IgG1 mAbs (REGN3470, REGN3471 and REGN3479) directed against different epitopes on Ebola virus glycoprotein
BIVV009 (sutimlimab)/Sanofi	Autoimmune hemolytic anemia	11/13/2020 FDA PDUFA date for humanized IgG4 mAb against total complement component 1
Lisocabtagene maraleucel/ Bristol-Myers Squibb	Diffuse large cell lymphoma	11/16/2020 FDA PDUFA date for autologous chimeric antigen receptor (CAR) modified T cells expressing a CD19 CAR and a truncated EGFR (EGFRt) that has no signaling capacity
Naxitamab/Y-mAbs	Brain cancer (malignant glioma, anaplastic astrocytoma, glioblastoma	11/30/2020 FDA PDUFA date for humanized IgG3 3F8 mAb targeting ganglioside GD2 and CD3
Inclisiran/Novartis (The Medicines Company)	Dyslipidemia/ hypercholesterolemia	12/1/2020 FDA PDUFA date for *N*-acetylgalactosamine (GalNAc)-conjugated 2′-*O*-methyl, 2′-fluoro, 2′-H-phosphorothioate siRNA-based oligonucleotide that targets *PCSK9* mRNA translation
Lumasiran/Alnylam	Hyperoxaluria	10/10/2020 EMA CHMP opinion on enhanced stabilization chemistry GalNAc-conjugated siRNA targeting glycolate oxidase
Valoctocogene roxaparvovec/ BioMarin Pharmaceutical	Hemophilia A	9/1/2020 EMA CHMP opinion on this AAV factor VIII gene therapy
PTC-AADC/PTC Therapeutics	Aromatic L-amino acid decarboxylase (AADC) deficiency	10/1/2020 EMA CHMP opinion on AAV delivering the human *DDC* gene, encoding AADC

PDUFA, Prescription Drug User Fee Act; CHMP, Committee for Medicinal Products for Human Use. Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com)

Notable regulatory setbacks (2Q20)

Drug/company	Indication	Drug information
Idecabtagene vicleucel/ Bristol-Myers Squibb and bluebird bio	Multiple myeloma	5/13/2020 FDA issued a refuse to file letter for this CAR-T therapy targeting B-cell maturation antigen as a result of missing information on the lentiviral vector and manufacturing processes
Nadofaragene firadenovec/FKD Therapies	Bladder cancer	5/31/2020 FDA issued a complete response letter with regard to this adenoviral recombinant interferon α2b gene therapy as a result of outstanding manufacturing issues
Abicipar pegol/ Allergan	Wet age-related macular degeneration	6/26/2020 FDA issued a complete response letter for this small-molecule designed ankyrin repeat protein (DARPIN) that binds all vascular endothelial growth factor isoforms, because of intraocular inflammation
Ocaliva/Intercept Pharmaceuticals	Non-alcoholic steatohepatitis	6/29/2020 FDA issued a complete response letter for an accelerated approval for this selective farnesoid X receptor (FXR) agonist because of unfavorable risk/ benefit ratio
LYS-SAF302/ Sarepta Therapeutics	Mucopolysaccharidosis IIIA (Sanfilippo A syndrome)	6/5/2020 FDA put a clinical hold on this AAV-10 carrying the human *N*-sulfoglucosamine sulfohydrolase (*SGSH*) cDNA following observations of localized findings on magnetic resonance imaging at the intracerebral injection sites
AT-132/Audentes Therapeutics	X-linked myotubular myopathy	6/5/2020 FDA put a clinical hold on this AAV-8 gene therapy technology carrying the *MTM1* gene because of two patient deaths

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