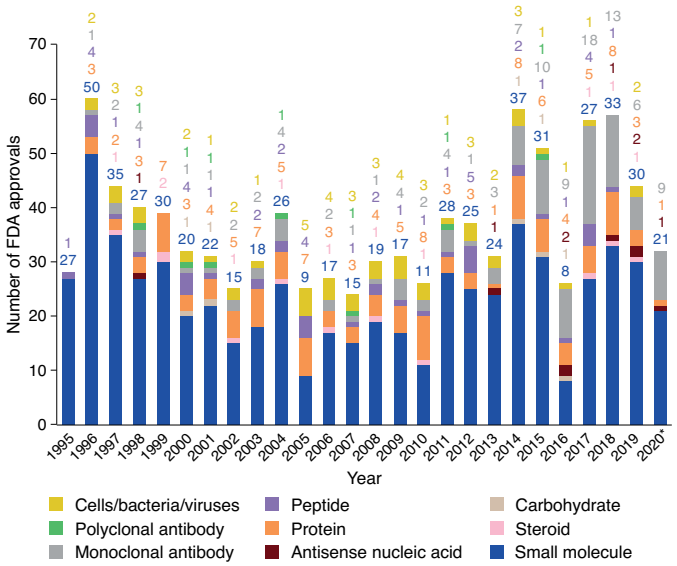
DATA PAGE

Drug pipeline 3Q20

US Food and Drug Administration (FDA) approvals in 3Q show no effect of the pandemic, with roughly the same number of approvals as in 3Q19. The agency granted three accelerated approvals for cancer indications, including two that target CD19 in unique ways (Gilead's Tecartus, which has circulating malignant cells removed during manufacturing, and MorphoSys's Monjuvi, which has modifications to the Fc portion). The first small-molecule exon-skipping drug, Roche's Evrysdi, was approved. The saga of Biogen's monoclonal aducanumab may finally come to a resolution in early 2021, when the FDA reviews data from its restarted continuation trial for Alzheimer's disease. Both the European Medicines Agency (EMA) and FDA gave thumbs down to BioMarin's hemophilia A drug valoctocogene roxaparvovec.

FDA approvals by drug type

Drug approvals stay the course, pandemic or no.



*Partial year through 30 September. Source: BioMedTracker, a service of Sagient Research (http://www.biomedtracker.com).

Notable clinical trial results (3Q20)

Drug/company	Indication	Drug information
TT11 CD30 CAR-T/Tessa Therapeutics	Hodgkin's lymphoma	7/23/2020 In a phase1/2 trial of CAR-T cells against CD-30 in heavily pretreated patients, 59% had complete response, 72% had overall response (*J. Clin. Oncol.* https://doi.org/10.1200/JCO.20.01342, 2020)
Nemolizumab/ Galderma	Atopic dermatitis	7/9/2020 In a phase 3 double-blind placebo controlled trial of this subcutaneous human monoclonal against IL-31AR, patients receiving the drug experienced greater reduction in pruritus than those receiving placebo (*N. Engl. J. Med.* **383**, 141–150, 2020)
Veklury (remdesivir)/ Gilead Sciences	COVID-19	8/21/2020 Patients randomized to a 5-day course of remdesivir had a statistically significant difference in clinical status compared with standard care (*JAMA* https://doi.org/10.1001/jama.2020.16349, 2020)

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

Notable drug approvals (3Q20)

Drug/company	Indication	Drug information
Tecartus (brexucabtagene autoleucel)/Gilead Sciences	Mantle cell lymphoma	7/24/2020 FDA grants accelerated approval for this CD19 chimeric antigen receptor (CAR)-T cell therapy from which circulating malignant cells are removed during manufacture
Monjuvi (tafasitamab)/ MorphoSys	Diffuse large cell lymphoma	7/31/2020 FDA grants accelerated approval for this humanized monoclonal antibody against the B-cell receptor CD19, with a modified Fc region to enhance antibody-dependent cellular toxicity
Blenrep (belantamab mafodotin)/ GlaxoSmithKline	Multiple myeloma	8/5/2020 FDA grants accelerated approval for this first-in-class antibody–drug conjugate, a humanized antibody directed against B cell maturation antigen (BCMA) and conjugated to auristatin F via a non-cleavable linker
Viltepso (viltolarsen)/ Nippon Shinyaku	Duchenne muscular dystrophy	8/12/2020 FDA approves this exon-53-specific antisense morpholino oligonucleotide
Gavreto (pralsetinib)/ Blueprint Medicines	Non-small-cell lung cancer	9/4/2020 FDA approves this small-molecule inhibitor of RET (rearranged during transfection) for patients with RET fusion mutations
Evrysdi (risdiplam)/ Roche	Spinal muscular atrophy	8/7/2020 FDA approves this small- molecule modulator of *SMN2* pre-mRNA splicing
Olinvyk (oliceridine)/ Trevena	Acute pain	8/7/2020 FDA approves this first-in-class GPCR inhibitor

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

Upcoming catalysts (1Q21)

Drug/company	Indication	Drug information
Aducanumab/Biogen	Alzheimer's disease	3/5/2021 FDA PDUFA date for this fully human IgG1 monoclonal against a conformational epitope on β-amyloid plaques
Idecabtagene vicleucel/Bristol Myers Squibb	Multiple myeloma	3/6/2021 FDA PDUFA date for this chimeric antigen receptor (CAR)-T cell targeting BCMA
Casimersen/Sarepta Therapeutics	Duchenne muscular dystrophy	2/25/2021 FDA PDUFA date for this phosphorodiamidate morpholino oligonucleotide that skips exon 45 of the mutated dystrophin
Tanezumab/Pfizer	Osteoarthritis and osteoarthritis pain	12/01/2020, 1/1/2021 FDA PDUFA and EMA CHMP (respectively) decisions on this anti-NGF IgG2Δa monoclonal antibody
Evinacumab/ Regeneron	Dyslipidemia / hypercholesterolemia	2/11/2021 FDA PDUFA date for this fully human monoclonal antibody made by VelocImmune technology and targeting angiopoietin-like-3 (ANGLPTL3)

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 (3Q20)

Drug/company	Indication	Drug information
Valoctocogene roxaparvovec/ BioMarin	Hemophilia A	8/18/2020, 9/9/2020 FDA and EMA ask for data going out to one (EMA) and two (FDA) years to show durable effect
Jyseleca/Gilead Sciences	Rheumatoid arthritis	8/18/2020 FDA issues a complete response letter for this small-molecule JAK1 inhibitor because of toxicity concerns and incomplete data from trial looking at effects on sperm production
UCARTCS1/ Cellectis	Multiple myeloma	7/6/2020 FDA issues clinical hold on this CAR-T therapy targeting a cell-surface protein overexpressed in multiple myeloma due to treatment-emergent cardiac arrest
Leronlimab/ CytoDyn	HIV/AIDS	7/13/2020 FDA issues refuse-to-file letter for this humanized IgG4 monoclonal antibody directed against CCR5 used in combination with antiviral therapy, citing not enough data for a substantive review
PBGM01/ Passage Bio	GM1 gangliosidosis	8/13/2020 FDA puts a clinical hold on this AAVhu68 capsid-delivered *GLB1* gene encoding lysosomal acid β-galactosidase due to biocompatibility of the proposed intra-cisterna magna delivery device

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