

2023년 FDA 신약 승인 현황

국가신약개발사업단 기획팀 진주연 연구원

Overview

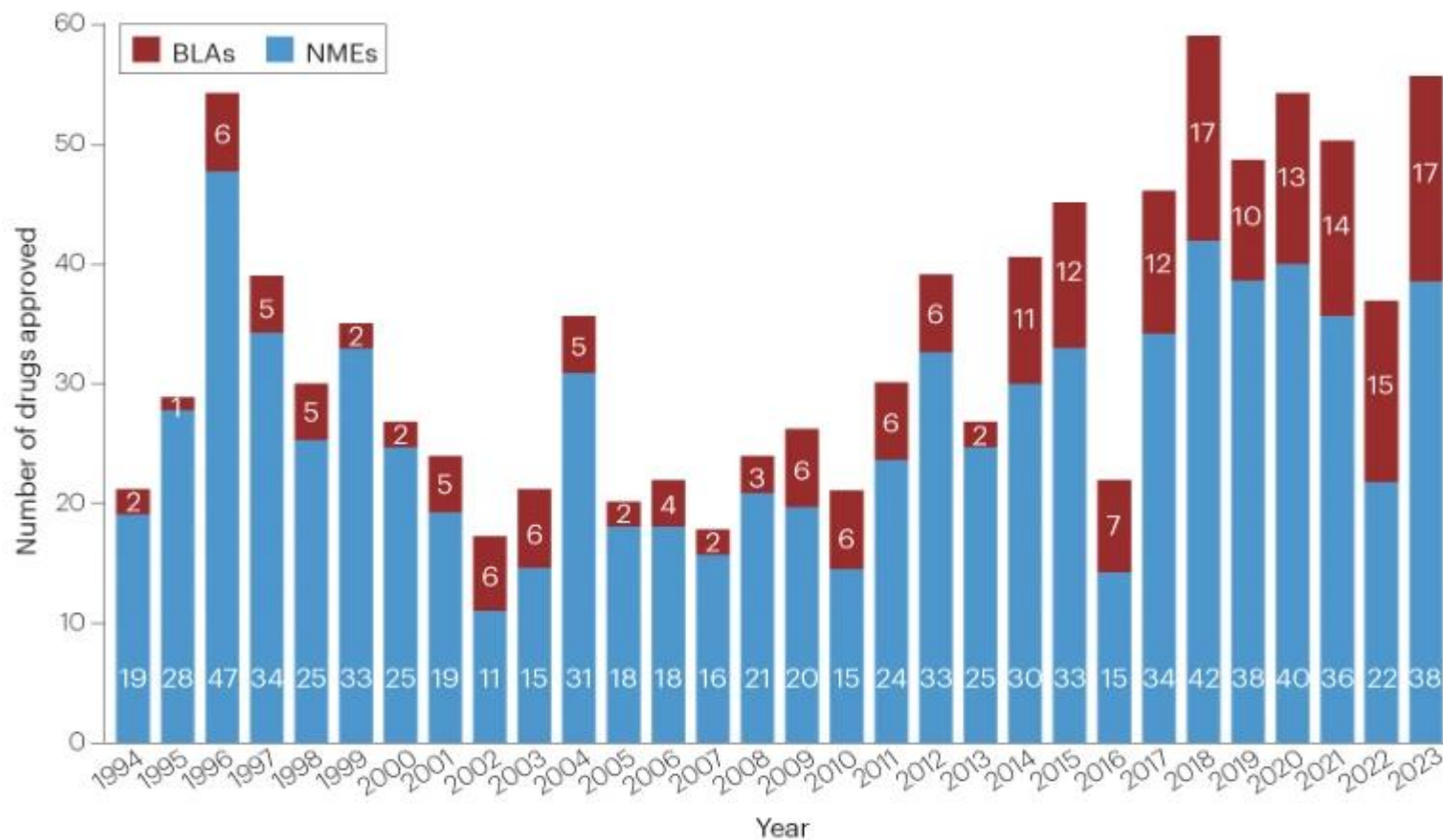
2023년 한 해 동안 미국 FDA는 총 55개의 신약을 승인했다(CDER 기준). 이는 2022년 승인 건수(총 37건) 대비 약 50% 증가한 수치다.

적응증 유형별로는 Oncology(13건), Neurology(9건), Infectious diseases & Haematology(각 5건)의 순으로 승인 되었으며, 모달리티 유형별로는 Small molecules(34건), Proteins(17건), Oligonucleotides(4건) 순으로 승인 되었다.

이번 자료에서는 네이처 리뷰지에서 공개한 2023년 승인 신약(55건)과 거절된 신약(9건), 올해의 주목할 만한 파이프라인(19건)을 소개한다.

1. 2023년 FDA 승인 현황

2023년 FDA는 의약품 평가연구센터(Center for Drug Evaluation and Research, CDER)에서 55개, 생물약품 평가연구센터(Center for Biologics Evaluation and Research, CBER)에서 16개로 총 71개의 신약을 승인하였다. 앞선 2022년의 FDA 승인 건수가 총 45건(CDER: 37, CBER: 8건)인 것에 비교하였을 때, 23년의 신약 승인은 크게 증가하였다(그림 1).

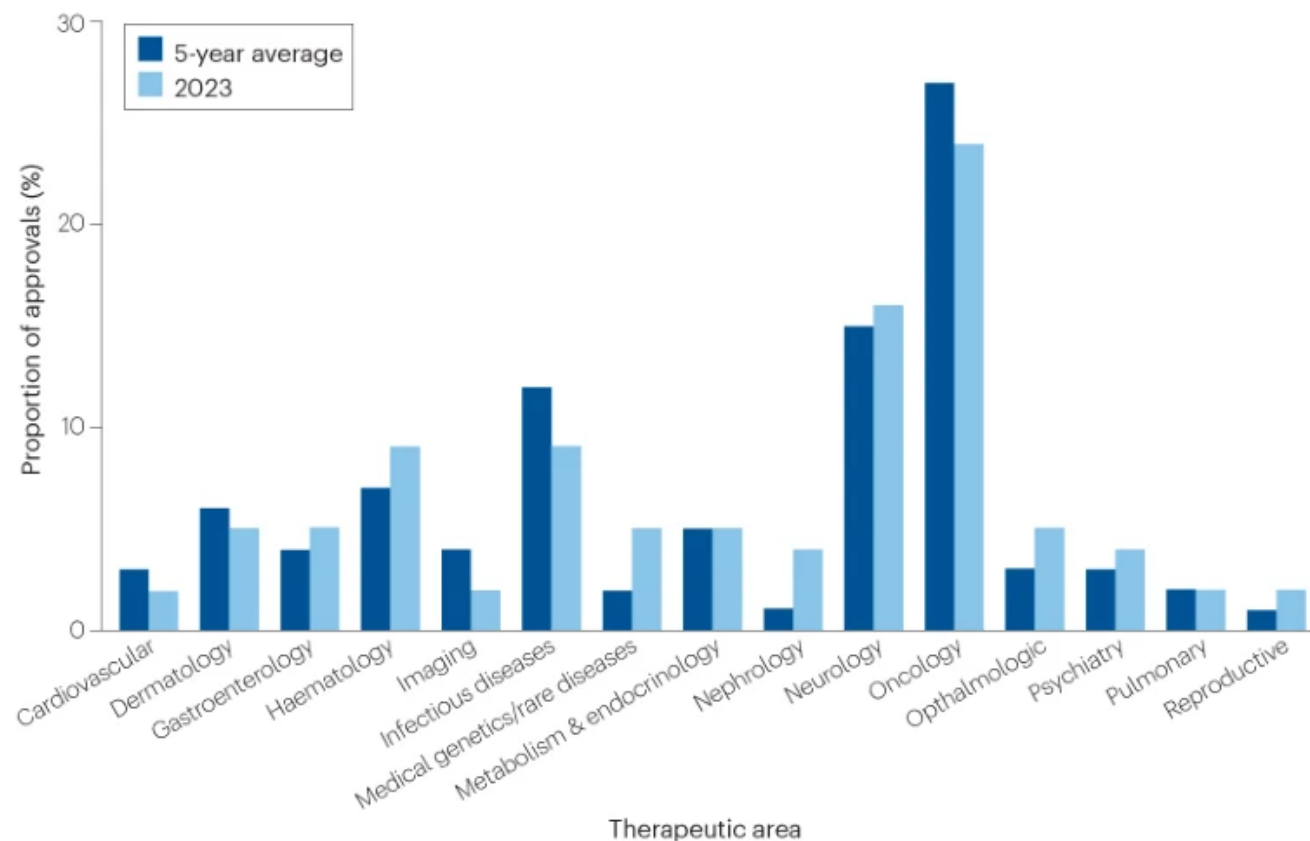


[그림 1] 연도별 FDA 승인 신약*(1994~2023)

*백신 및 유전자 요법을 포함하여 CBER에서 승인한 제품은 포함되지 않음

** BLAs: Biological License Applications(생물의약품 품목허가), NMEs: New Molecular Entities(신규물질 품목허가)

※ 출처: A Mullard, "2023 FDA approvals", Nature reviews drug discovery, 2024.



[그림 2] 적응증 유형별 승인 현황

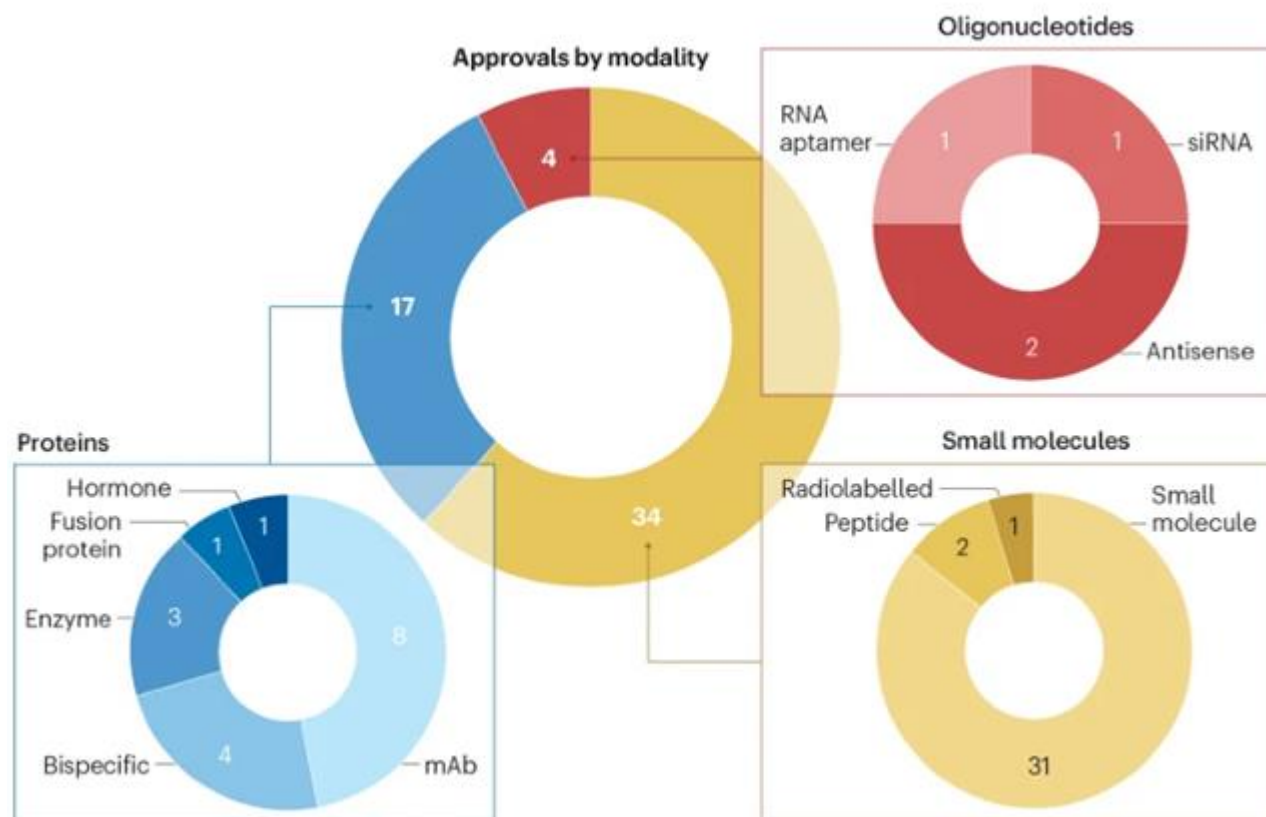
승인 내역(CDER 기준)을 적응증 유형별로 살펴보았을 때, 암 치료제(Oncology)가 13건 (24%) 승인되어 가장 큰 비중을 차지하고 있으며, 신경 질환(Neurology)은 9건(16%)으로 두 번째로 많은 승인을 받았다.

감염성 질환(Infectious diseases)과 혈액 질환(Haematology)은 각각 5건(9%)씩 승인을 받았다(그림 2).

* 복수의 적응증에 해당하는 질병영역은 하나로 분류되었음

** 5-year average: 2018~2022년 평균

※ 출처: A Mullard, "2023 FDA approvals", Nature reviews drug discovery, 2024.



모달리티 유형별로는 저분자 신약(Small molecules)이 총 34 건(62%)으로 가장 큰 비중을 차지 하였으며, RNA 압타머 제품 승인(1 건)을 포함한 올리고뉴클레오타이드(Oligonucleotides) 치료제 승인은 총 4 건(7%)이었다(22 년 올리고뉴클레오타이드 치료제 1 건 승인).

단일항체 8 건(15%)과 이중항체 4 건(7%) 등을 포함한 단백질 신약(Proteins)은 총 17 건(31%)이 승인 되었다(그림 3).

[그림 3] 모달리티 유형별 CDER 승인 현황

※ 출처: A Mullard, "2023 FDA approvals", Nature reviews drug discovery, 2024.

2. 2023년 FDA 승인 거절 내역(9개)

2023년에 승인 거절된 약물은 총 9개로 (1) Donanemab(Eli Lilly), (2) Omecamtiv mecarbil(Cytokinetics), (3) Inbakicept(ImmunityBio), (4) Concizumab(Novo Nordisk), (5) Obeticholic acid(Alfasigma), (6) Zuranolone(Sage), (7) Avasopasem manganese(Galera), (8) Lebrikizumab(Eli Lilly), (9) Gefapixant(Merck & Co.)이다. Zuranolone은 기존에 신청한 주요 우울 장애(MDD, Major Depressive Disorder)로는 거절되었으나 산후우울증 치료제(MDD, Major Depressive Disorder)로 승인(23.8.4.)을 받은 바 있다.

<표 1> 2023년 FDA 승인 거절 내역

연번	성분명(제품명)	기업명	작용기전	적응증
1	Donanemab	Eli Lilly	Amyloid-β-targeted mAb	Alzheimer disease
2	Omecamtiv mecarbil	Cytokinetics	Myosin activator	Chronic heart failure
3	Inbakicept	ImmunityBio	IL-15 agonist fusion protein	Bladder cancer
4	Concizumab	Novo Nordisk	TFPI-targeted mAb	Haemophilia A and B
5	Obeticholic acid (Ocaliva) ¹⁾	Alfasigma	Farnesoid X receptor agonist	NASH
6	Zuranolone (Zurzuvae) ²⁾	Sage	GABAA receptor PAM	Major Depressive Disorder
7	Avasopasem manganese	Galera	Superoxide dismutase mimetic	Mucositis
8	Lebrikizumab	Eli Lilly	IL-13-targeted mAb	Atopic dermatitis
9	Gefapixant	Merck & Co.	P2X3 receptor antagonist	Chronic cough

1) 2016년 원발성 담즙성 담관염 치료제로 승인

2) 2023년 산후우울증 치료제로 승인

※ 출처: A Mullard, "2023 FDA approvals", Nature reviews drug discovery, 2024.

3. 2024년 주목할 만한 파이프라인(19개)

네이처 리뷰지에서 소개한 올해의 주목할 만한 파이프라인은 19개이다.

<표 2> 2024년 주목할 만한 파이프라인

연번	성분명(제품명)	기업명	작용기전	적응증	승인 예상 시기
1	Zolbetuximab	Astellas	Claudin 18.2-targeted mAb	Gastric cancer	January
2	Lifileucel	Iovance	Tumour-infiltrating lymphocyte therapy	Melanoma	February
3	Resmetirom	Madrigal/Synta	Thyroid hormone receptor β agonist	NASH	March
4	Sotatercept	Merck & Co./Acceleron	Fusion protein ligand trap for TGF- β superfamily	PAH	March
5	mRNA-1345	Moderna	mRNA-based vaccine	RSV prevention	April
6	Donanemab	Eli Lilly	Amyloid- β -targeted mAb	Alzheimer disease	Q1
7	EB-101	Abeona	Gene therapy with COL7A2 transgene	RDEB	May
8	Patritumab deruxtecan	Merck & Co.	HER3-targeted ADC	NSCLC	June
9	Imetelstat	Geron	Telomerase inhibitor	Transfusion-dependent anaemia with MDS	June
10	Tarlatamab	Amgen	DLL3 \times CD3 T-cell engager antibody	SCLC	June
11	Fidanacogene elaparvovec	Pfizer/Spark	AAV-based gene therapy with factor IX transgene	Hemophilia B	Q2
12	Bentracimab	Laboratoires SERB	Ticagrelor-neutralizing antibody	Drug toxicity	1H
13	Crovalimab	Roche	C5-targeted mAb	PNH	July
14	Danicopan	AstraZeneca/Alexion	Factor D inhibitor	PNH	July
15	Midomafetamine	MAPS	MDMA	PTSD	August
16	Xanomeline plus trospium	Karuna/BMS	Muscarinic receptor modulators	Schizophrenia	September
17	Acoramidis	BridgeBio	TTR stabilizer	TTR amyloidosis	December
18	Marstacimab	Pfizer	TFPI-targeted mAb	Haemophilia A and B	Q4
19	Afamitresgene autoleucel	Adaptimmune	MAGE-A4-targeted autologous, engineered T cell therapy	Synovial sarcoma	2024

※ 출처: A Mullard, "2023 FDA approvals", Nature reviews drug discovery, 2024.

[참고] 2023년 FDA 승인 리스트

<2023년 FDA CDER 승인 내역>

연번	성분명(제품명)	기업명	작용기전	적응증
1	Lecanemab (Leqembi)	Eisai/Biogen	Amyloid- β -targeted mAb	Alzheimer disease
2	Bexagliflozin (Brenzavvy)	Theracosbio	SGLT2 inhibitor	Glycaemic control in type 2 diabetes mellitus
3	Pirtobrutinib (Jaypirca)	Loxo/Eli Lilly	BTK inhibitor	Mantle cell lymphoma
4	Elacestrant (Orserdu)	Stemline	ER antagonist	ER-positive, HER2-negative, ESR1-mutant breast cancer
5	Daprodustat (Jesduvroq)	GSK	HIF-PH inhibitor	Anaemia caused by CKD for adults on dialysis
6	Velmanase alfa (Lamzede)	Chiesi	Recombinant α -mannosidase	Non-CNS manifestations of α -mannosidosis
7	Sparsentan (Filspari)	Travere	Endothelin and angiotensin II receptor antagonist	Proteinuria in primary IgA nephropathy
8	Omaveloxolone (Skyclarys)	Reata/Biogen	Mechanism unknown, NRF2 activator	Friedrich's ataxia
9	Zavegepant (Zavzpret)	Pfizer	CGRP receptor antagonist	Migraine
10	Trofinetide (Daybue)	Acadia	Mechanism unknown	Rett syndrome
11	Retifanlimab (Zynzyza)	Incyte	PD1-targeted mAb	Merkel cell carcinoma
12	Rezafungin (Rezzayo)	Cidara	Echinocandin antifungal	Candidemia and invasive candidiasis
13	Leniolisib (Joenja)	Pharming	PI3K δ inhibitor	Activated PI3K δ syndrome
14	Tofersen (Qalsody)	Biogen	SOD1-targeted ASO	SOD1 amyotrophic lateral sclerosis
15	Pegunigalsidase alfa (Elfabrio)	Chiesi	PEGylated recombinant α -galactosidase A	Fabry disease
16	Fezolinetant (Veozah)	Astellas	Neurokinin 3 receptor antagonist	Hot flashes caused by menopause
17	Perfluorohexyloctane (Miebo)	Bausch + Lomb	Semifluorinated alkane	Dry eye disease
18	Epcoritamab (Epkiny)	Genmab/AbbVie	CD20 \times CD3 T-cell engager	DLBCL and high-grade B-cell lymphoma
19	Sulbactam, durlobactam (Xacduro)	Entasis	β -lactam antibacterial plus a β -lactamase inhibitor	Hospital-acquired and ventilator-associated bacterial pneumonia caused by susceptible ABC
20	Nirmatrelvir, ritonavir (Paxlovid)	Pfizer	SARS-CoV-2 main protease inhibitor plus a CYP3A	Mild-to-moderate COVID-19

연번	성분명(제품명)	기업명	작용기전	적응증
			inhibitor	
21	Flotufolastat F18 (Posluma)	Blue Earth	Radioactive diagnostic agent	PET imaging in prostate cancer
22	Sotagliflozin (Inpefa)	Lexicon	SGLT1/2 inhibitor	Heart failure
23	Glofitamab (Columvi)	Genentech	CD20 × CD3 T-cell engager	DLBCL or large B-cell lymphoma
24	Ritlecitinib (Litfulo)	Pfizer	JAK3 inhibitor	Alopecia areata
25	Rozanolixizumab (Rystiggo)	UCB	FcRn-targeted mAb	AChR- or MuSK-antibody-positive gMG
26	Somatrogon (Ngenla)	Pfizer	Human growth hormone analogue	Growth hormone deficiency
27	Nirsevimab (Beyfortus)	AstraZeneca	RSV F protein-targeted mAb	RSV lower respiratory tract disease
28	Quizartinib (Vanflyta)	Daiichi Sankyo	FLT3 kinase inhibitor	AML
29	Lotilaner (Xdemvy)	Tarsus	Ectoparasiticide	Demodex blepharitis
30	Zuranolone (Zurzuvae)	Sage	GABAA receptor PAM	Postpartum depression
31	Avacincaptad pegol (Izervay)	Iveric/Astellas	C5-targeted aptamer	Geographic atrophy secondary to AMD
32	Talquetamab (Talvey)	Janssen	GPRC5D × CD3 T-cell engager	Multiple myeloma
33	Elranatamab (Elrexfio)	Pfizer	BCMA × CD3 T-cell engager	Multiple myeloma
34	Palovarotene (Sohonos)	Ipsen	Retinoic acid receptor agonist	Fibrodysplasia ossificans progressiva
35	Pozelimab (Veopoz)	Regeneron	C5-targeted mAb	CHAPLE disease
36	Motixafortide (Aphexda)	BiolineRx	CXCR4 inhibitor	Hematopoietic stem cell mobilization for autologous transplantation in multiple myeloma
37	Momelotinib (Ojjaara)	GSK	JAK1/2, ALK2 inhibitor	Myelofibrosis in adults with anaemia
38	Gepirone (Exxua)	Fabre-Kramer	5HT1A receptor agonist	Major depressive disorder
39	Cipaglucosidase alfa (Pombiliti)	Amicus	Recombinant α-glucosidase	Pompe disease
40	Nedosiran (Rivfloza)	Novo Nordisk	LDHA-targeted siRNA	Primary hyperoxaluria type 1
41	Etrasimod (Velsipity)	Pfizer	S1P receptor modulator	Ulcerative colitis
42	Zilucoplan (Zilbrysq)	UCB	Complement C5 inhibitor	AChR-antibody positive gMG
43	Bimekizumab (Bimzelx)	UCB	IL-17A/F-targeted mAb	Plaque psoriasis

연번	성분명(제품명)	기업명	작용기전	적응증
44	Vamorolone (Agamree)	Santhera	Corticosteroid	Duchenne muscular dystrophy
45	Mirikizumab (Omvoh)	Eli Lilly	IL-23-targeted mAb	Ulcerative colitis
46	Toripalimab (Loqtorzi)	Coherus	PD1-targeted mAb	Nasopharyngeal carcinoma
47	Fruquintinib (Fruzaqla)	Takeda	VEGFR1/2/3 kinase inhibitor	Colorectal cancer
48	Taurolidine, heparin (Defencath)	Cormedix	Thiadiazinane antimicrobial plus an anticoagulant	Incidence of catheter-related bloodstream infections
49	Repotrectinib (Augtyro)	Bristol Myers Squibb	ROS1 and TRK kinase inhibitor	ROS1-positive NSCLC
50	Efbemalenograstim alfa (Ryzneuta)	Evive	Recombinant leukocyte growth factor	Neutropenia
51	Capivasertib (Truqap)	AstraZeneca	AKT kinase inhibitor	Breast cancer
52	Nirogacestat (Ogsiveo)	Springworks	γ -secretase inhibitor	Desmoid tumours
53	Iptacopan (Fabhalta)	Novartis	Complement factor B inhibitor	Paroxysmal nocturnal haemoglobinuria
54	Birch triterpenes (Filsuvez)	Chiesi	Mechanism unknown	Epidermolysis bullosa
55	Eplontersen (Wainua)	Ionis/AstraZeneca	TTR-targeted ASO	hATTR with polyneuropathy

※ 출처: A Mullard, "2023 FDA approvals", Nature reviews drug discovery, 2024.

<2023년 FDA CBER 승인 내역>

연번	성분명(제품명)	기업명	작용기전	적응증
1	Fc-vwf-xten fusion protein (Altuviio)	Bioerativ	Recombinant antihemophilic factor	Haemophilia A
2	Omidubicel (Omisirge)	Gamida Cell	Nicotinamide modified allogeneic haematopoietic progenitor cell therapy	Neutrophil recovery in patients with haematologic malignancies
3	Fecal microbiota spores, live (Vowst)	Seres	Bacterial spore suspension in capsules for oral administration	C. difficile recurrence
4	RSV vaccine, adjuvanted (Arexvy)	GSK	Stabilized prefusion F subunit vaccine	RSV prevention, in over 60s
5	Beremagene geperpavec (Vyjuvek)	Krystal	HSV-based gene therapy with OL7A1 transgene	Skin wounds in COL7A1-mutated DEB
6	RSV vaccine (Abrysvo)	Pfizer	Bivalent stabilized prefusion F subunit vaccine	RSV prevention, in over 60s and pregnant women
7	Delandistrogene moxeparvovec (Elevidys)	Sarepta	AAV-based gene therapy with a micro-dystrophin transgene	DMD patients aged 4–5
8	Donislecel (Lantidra)	Celltrans	Allogeneic pancreatic islets	Type 1 diabetes patients unable to approach target HbA1c
9	Valoctocogene roxaparvovec (Roctavian)	Biomarin	AAV-based gene therapy with modified factor VIII transgene	Haemophilia A
10	Anthrax vaccine adsorbed, adjuvanted (Cyfendus)	Emergent	Protective antigen protein-based vaccine	Post-exposure prophylaxis
11	Prothrombin complex concentrate, human (Balfaxar)	Octapharma	Prothrombin complex concentrate containing factor II, VII, IX and X, and proteins C and S	Urgent warfarin reversal
12	Meningococcal groups A, B, C, W, and Y vaccine (Penbraya)	Pfizer	Conjugate and recombinant protein vaccine, Pentavalent	Meningococcal disease prevention
13	Chikungunya vaccine, live (Ixchiq)	Valneva	Live, attenuated chikungunya virus vaccine	Chikungunya virus prevention
14	ADAMTS13, recombinant (Adzynma)	Takeda	Recombinant rADAMTS13	cTTP
15	Exagamglogene autotemcel (Casgevy)	Vertex/CRISPR	CRISPR–Cas9-based BCL11a-gene-editing therapy	Sickle cell disease
16	Lovotibeglogene autotemcel (Lyfgenia)	Bluebird	Lentivirus-based gene therapy with HbAT87Q transgene	Sickle cell disease

※ 출처: A Mullard, "2023 FDA approvals", Nature reviews drug discovery, 2024.

<문의>

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Reference

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