



KDDF

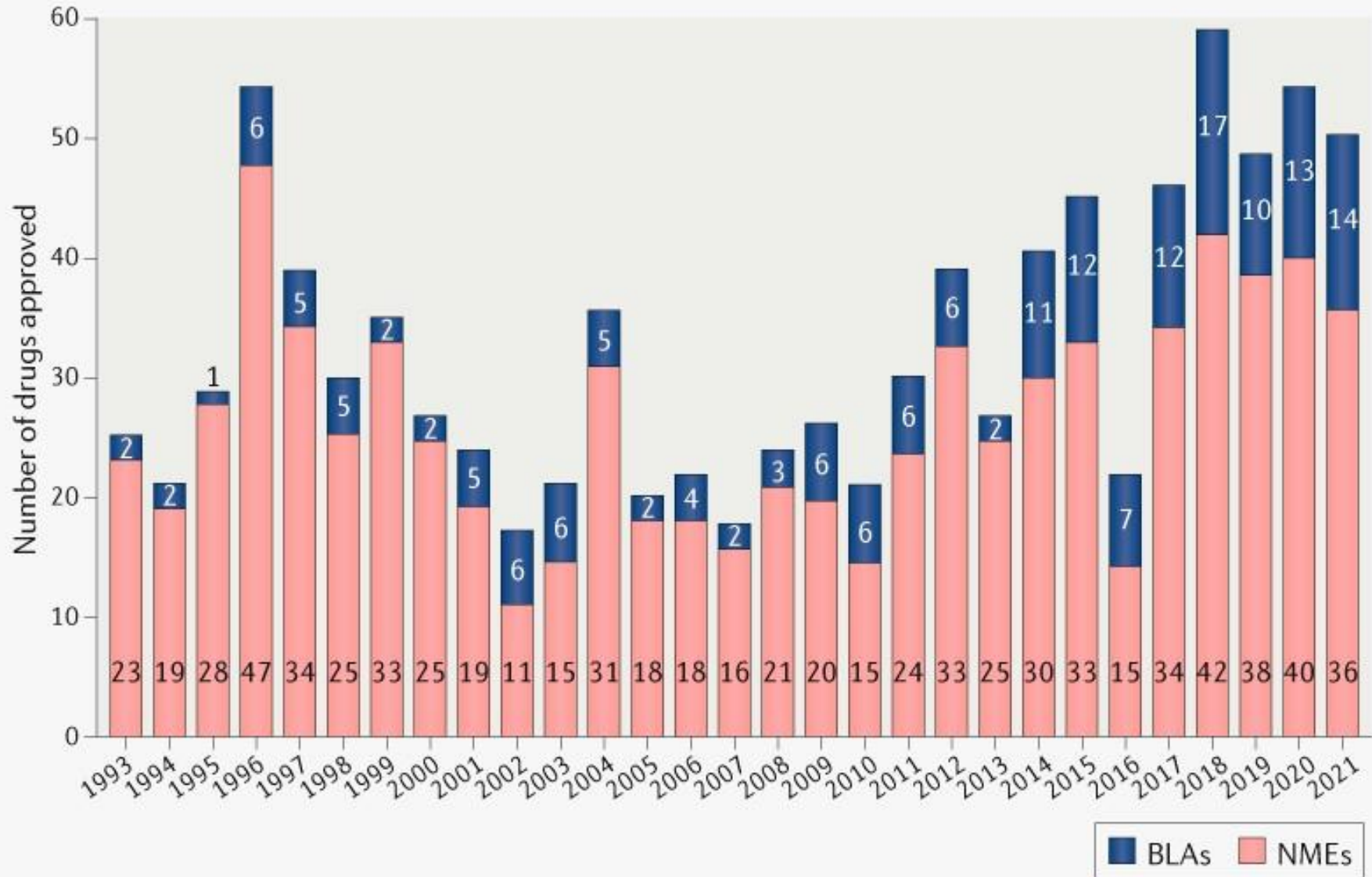
국가신약개발재단

Korea Drug Development Fund

2021년 FDA 승인 신약

기획운영팀
선임연구원 곽서연

2021년 FDA 승인 신약

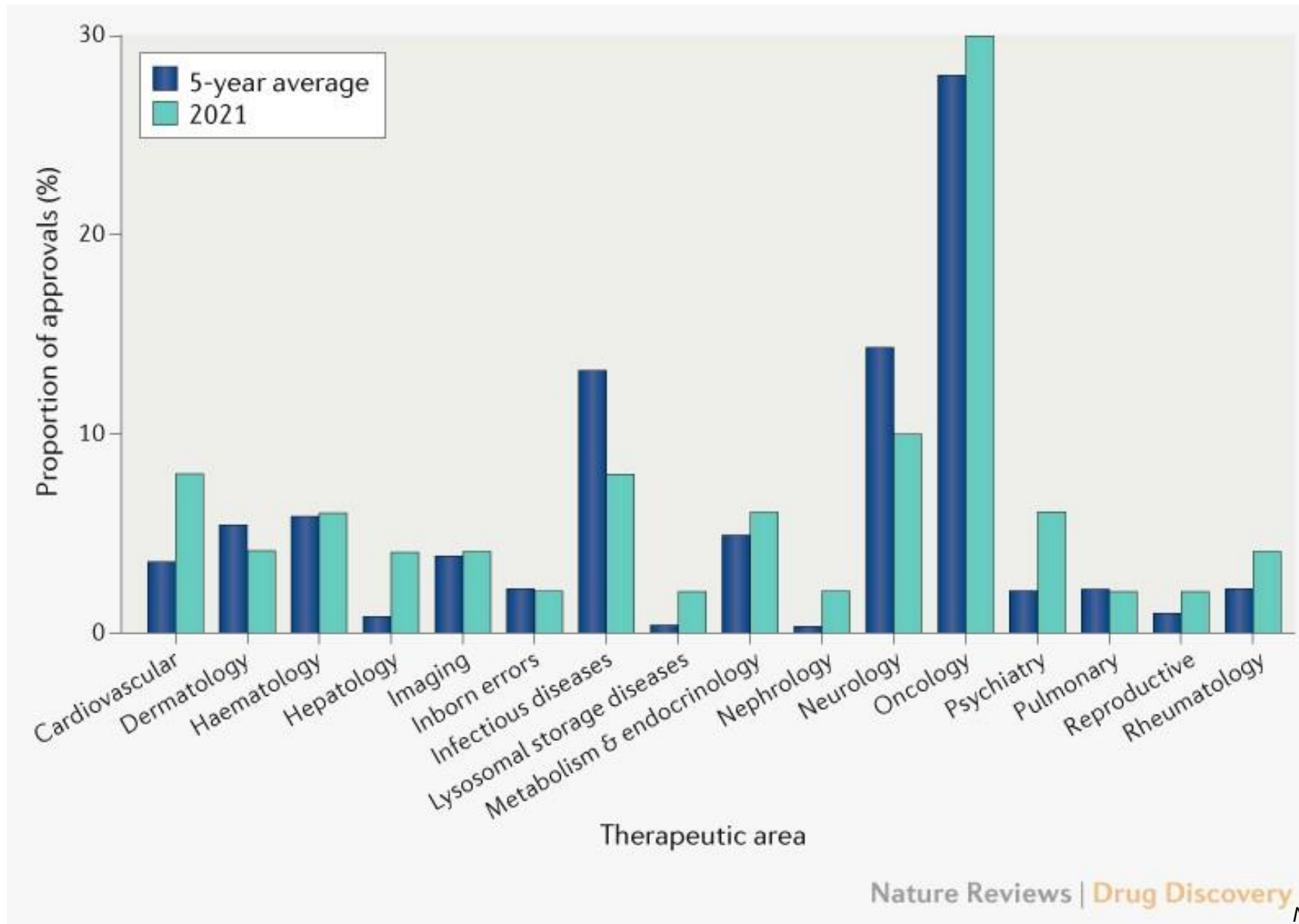


BLAs:
Biologics License Applications

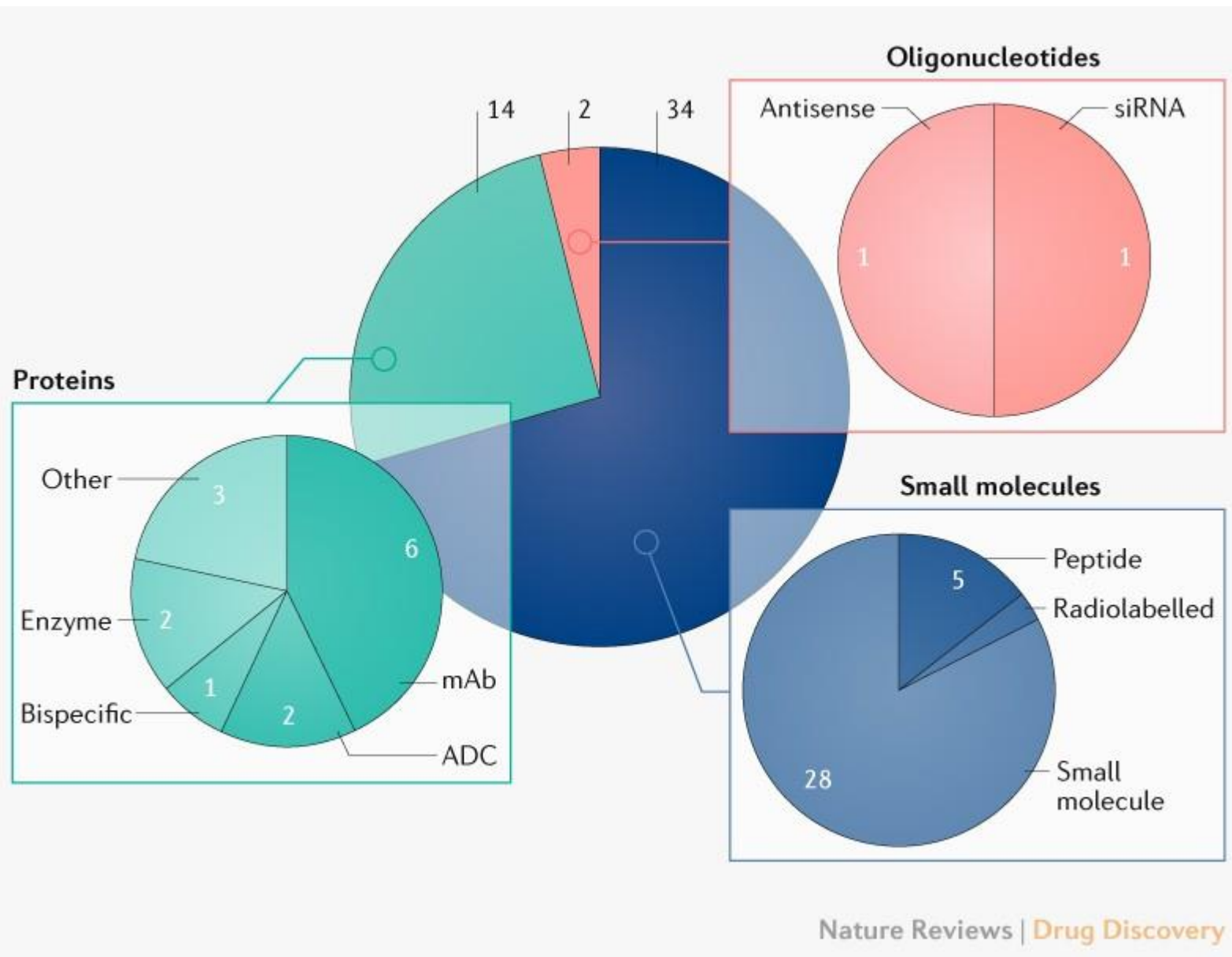
NMEs:
New Molecular Entities

- 2021년 FDA가 승인한 신약은 **총 50개** (백신과 유전자/세포치료제 미포함)
- 2021년 FDA 승인 신약 중 주목할 만한 신약:
 - 첫 KRAS inhibitor 항암제 (Amgen, Lumakras)
 - 첫 anti-amyloid antibody 알츠하이머 치료제 (Biogen, Aduhelm)
- 2021년 첫 FDA 승인 신약을 보유하게 된 회사:
 - Apellis, BridgeBio, ChemoCentryx
- 2개 이상의 신약을 허가 받은 회사:
 - AstraZeneca, BridgeBio, J&J, Merck, Novartis, Sanofi, and Takeda

2021년 FDA 승인 신약_적응증별

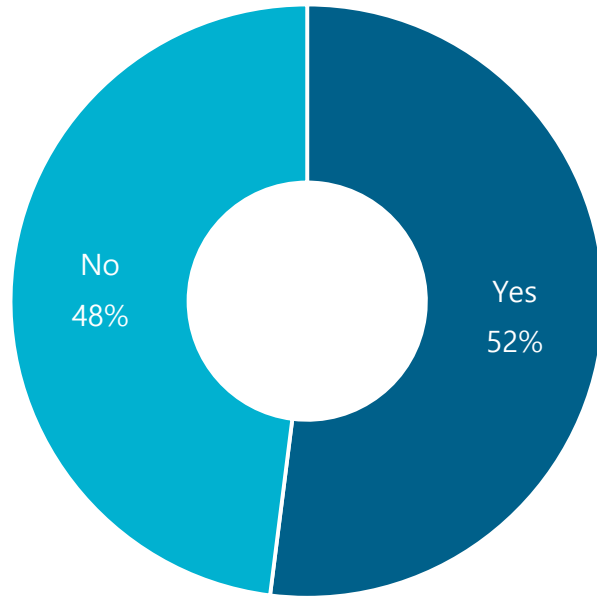


- 2021년 FDA 승인 신약 비율 중
 - **종양신약**이 가장 많은 비중(30%)을 차지하였는데, 이는 최근 5년 평균보다 증가한 수치임
 - 더불어, 심혈관질환, 혈액질환, 정신과질환 신약이 상대적으로 큰 폭으로 증가함
 - 신경과질환과 감염성 질환 신약은 각각 10%와 8%를 차지하며,
2번째와 3번째로 많은 비중을 차지하였으나, 5년 평균에 비하여 크게 감소한 수치임

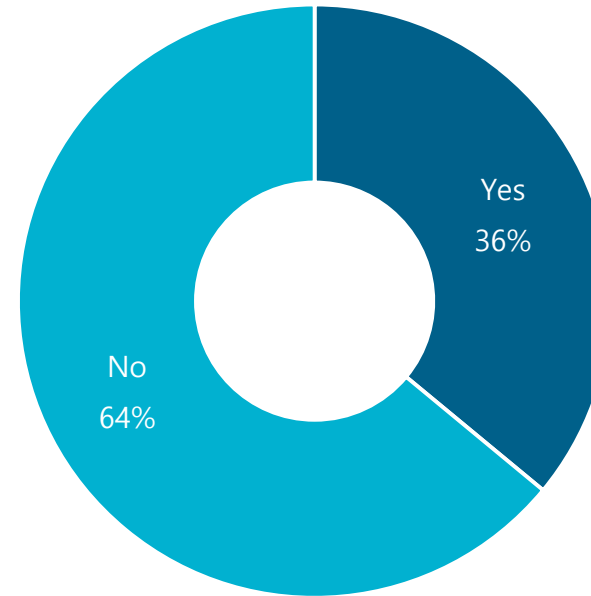


- 저분자 신약이 여전히 신약의 대부분을 차지함
- 새로운 ADC 신약:
Tivdak (Seagen/Genmab),
Tissue-factor-directed ADC
- 이중항체 신약:
Amivantamab(Rybrevant),
EGFR x METR bispecific mAb

Orphan Drug

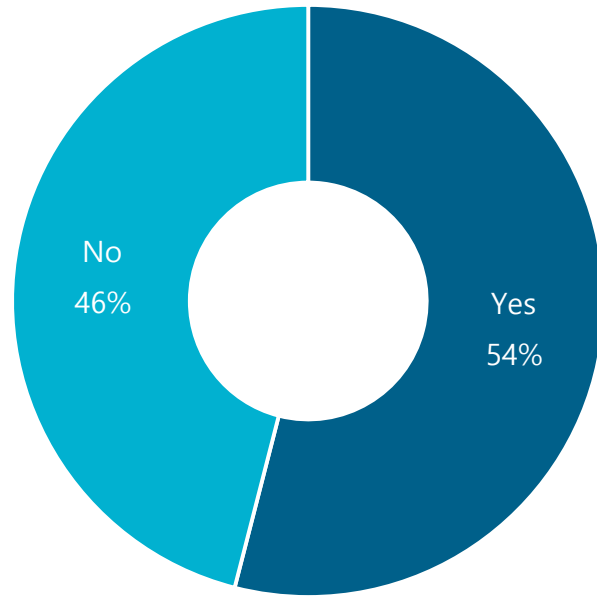


Fast Track

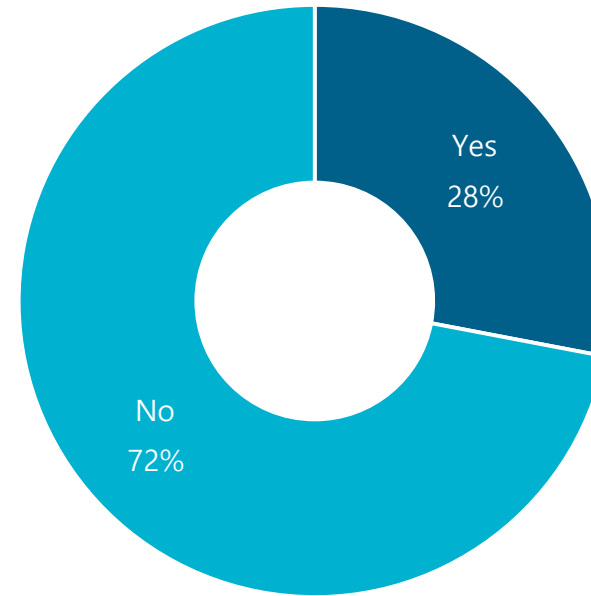


- 희귀질환신약 비중은 2020년(58.4%)과 비교하여 다소 감소
- Fast Track 승인 비중은 2020년(32%)과 비교하여 다소 증가

First-in-Class



Breakthrough Therapies



- First-in-Class 신약들의 승인율은 2020년(39.6%)과 비교하여 크게 증가
- Breakthrough Therapies는 2020년(41.5%)과 비교하여 크게 감소하였으나, 2019년 (27.0%), 2018년 (23.7%)과 비교하면 비슷한 수치임

- Medical Breakthrough (특히 cancer) 신약들과 '최초' 치료제의 경우,
더 잘 승인해주는 경향이 있음
 - Aduhelm (Biogen)
- 이미 다른 대안 치료제가 존재하는 경우에는 보수적인 경향을 보임
 - Roxadustat (Fibrogen)
- 최초로 치료제가 나온 희귀질환들:
 - Pruritus: Bylvay(Albireo), Livmarli(Mirum)
 - Molybdenum cofactor deficiency type A: Nulibry(BridgeBio)

2021년 FDA 승인 신약 I

Sponsor	Trade name	Drug	Properties	Indication	Dosage Form	First-in-Class	Orphan Drug
AbbVie	Qulipta	Atogepant	CGRP receptor antagonist	Episodic migraine	Tablet		
ADC Therapeutics	Zynlonta	Loncastuximab tesirine	CD19-targeted ADC	B-cell lymphoma	Injection	Y	Y
Albireo	Bylvay	Odevixibat	IBAT inhibitor	Pruritus in PFIC	Capsule/Oral Pellet	Y	Y
Alkermes	Lybalvi	Olanzapine; samidorphan	Atypical antipsychotic and opioid antagonist	Schizophrenia and bipolar I disorder	Tablet		
Amgen	Lumakras	Sotorasib	KRAS-G12C inhibitor	KRASG12C-mutated NSCLC	Tablet	Y	Y
Apellis	Empaveli	Pegcetacoplan	Complement protein C3 inhibitor	PNH	Injection	Y	Y
Argenx	Vyvgart	Efgartigimod alfa	FcRn-binding Fc fragment	Myasthenia gravis	Injection	Y	Y
Ascendis Pharma	Skytrofa	Lonapegsomatropin	PEGylated human growth hormone	Growth failure due to GHD	Injection		Y
AstraZeneca	Saphnelo	Anifrolumab	IFNAR-targeted mAb	SLE	Injection	Y	
AstraZeneca/Amgen	Tezspire	Tezepelumab	TSLP-targeted mAb	Severe asthma	Injection	Y	
Aurinia	Lupkynis	Voclosporin	Calcineurin inhibitor	Lupus nephritis	Capsule	Y	
Aveo	Fotivda	Tivozanib	VEGFR kinase inhibitor	Renal cell carcinoma	Capsule		
Bayer	Kerendia	Finerenone	Non-steroidal MR antagonist	CKD with type 2 diabetes	Tablet	Y	

2021년 FDA 승인 신약 II

Sponsor	Trade name	Drug	Properties	Indication	Dosage Form	First-in-Class	Orphan Drug
Biogen/Eisai	Aduhelm	Aducanumab	Amyloid-β-targeted mAb	Alzheimer's disease	Injection	Y	
Biomarin	Voxzogo	Vosoritide	CNP analogue	Achondroplasia	Injection	Y	Y
BridgeBio	Nulibry	Fosdenopterin	cPMP	MoCD type A	Injection	Y	Y
BridgeBio	Truseltiq	Infigratinib	FGFR2 kinase inhibitor	FGFR2-mutated bile duct cancer	Capsule		Y
Cara Therapeutics	Korsuva	Difelikefalin	κ-Opioid receptor agonist	Pruritus associated with CKD	Injection	Y	
ChemoCentryx	Tavneos	Avacopan	Complement 5a receptor antagonist	ANCA-associated vasculitis	Capsule	Y	Y
Commave Therapeutics	Azstarys	Dexamethylphenidate; serdexmethylphenidate	CNS stimulant	ADHD	Capsule		
EMD Serono	Tepmetko	Tepotinib	MET kinase inhibitor	NSCLC	Tablet		Y
G1 Therapeutics	Cosela	Trilaciclib	CDK4 and CDK6 kinase inhibitor	Chemotherapy-induced myelosuppression	Injection	Y	
GlaxoSmithKline	Jemperli	Dostarlimab	PD1-targeted mAb	Endometrial cancer	Injection		
J&J	Ponvory	Ponesimod	S1P receptor modulator	Relapsing multiple sclerosis	Tablet		
J&J	Rybrevant	Amivantamab	EGFR×METR bispecific antibody	EGFR exon 20-mutated NSCLC	Injection	Y	
Jazz	Rylaze	Asparaginase erwinia chrysanthemi	Recombinant asparagine-specific enzyme	ALL and LBL, in patients allergic to E. coli-derived products	Injection		Y

2021년 FDA 승인 신약 III

Sponsor	Trade name	Drug	Properties	Indication	Dosage Form	First-in-Class	Orphan Drug
Kadmon	Rezurock	Belumosudil	ROCK2 kinase inhibitor	Chronic GVHD	Tablet	Y	Y
LEO Pharma	Adbry	Tralokinumab	IL-13-targeted mAb	Atopic dermatitis	Injection	Y	
Mayne Pharma	Nextstellis	Drospirenone; estetrol	Spirolactone and estrogen analogues	To prevent pregnancy	Tablet		
Merck & Co.	Welireg	Belzutifan	HIF-2 α inhibitor	von Hippel-Lindau disease	Tablet	Y	Y
Merck & Co./Bayer	Verquvo	Vericiguat	sGC stimulator	Chronic heart failure	Tablet	Y	
Mirum	Livmarli	Maralixibat	IBAT inhibitor	Pruritus in Alagille syndrome	Oral Solution		Y
Novartis	Scemblix	Asciminib	ABL/BCR-ABL1 kinase inhibitor	Ph+ CML	Tablet		Y
Novartis/Alnylam	Leqvio	Inclisiran	PCSK9-targeted siRNA	HeFH or ASCVD	Injection	Y	
On Target Labs	Cytalux	Pafolacianine	Fluorescent FR imaging agent	Ovarian cancer imaging	Injection	Y	Y
Oncopeptides	Pepaxto	Melphalan flufenamide	Peptide-conjugated alkylating drug	Multiple myeloma	Injection		Y
Pharmaessentia	Besremi	Ropeginterferon alfa-2b	PEGylated interferon α -2b	Polycythaemia vera	Injection	Y	Y
Progenics	Pylarify	Piflufolastat F-18	Radio-labelled PSMA imaging agent	Prostate cancer imaging	Injection		
Regeneron	Evkeeza	Evinacumab	ANGPTL3-targeted mAb	HoFH	Injection	Y	Y

2021년 FDA 승인 신약 IV

Sponsor	Trade name	Drug	Properties	Indication	Dosage Form	First-in-Class	Orphan Drug
Sanofi	Nexviazyme	Avalglucosidase alfa	Recombinant α -glucosidase	Pompe disease	Injection		Y
Sanofi/DNDi	Fexinidazole	Fexinidazole	Nitroimidazole antimicrobial	Sleeping sickness	Tablet		Y
Sarepta	Amondys45	Casimersen	Exon 45-skipping ASO	DMD	Injection		Y
Scynexis	Brexafemme	Ibrexafungerp	Triterpenoid antifungal	Vulvovaginal candidiasis	Tablet	Y	
Seagen/Genmab	Tivdak	Tisotumab vedotin	Tissue-factor-directed ADC	Cervical cancer	Injection	Y	
Supernus	Qelbree	Viloxazine	SNRI	ADHD	Capsule		
Takeda	Exkivity	Mobocertinib	EGFR kinase inhibitor	EGFR exon 20-mutated NSCLC	Capsule		Y
Takeda	Livtency	Maribavir	CMV pUL97 kinase inhibitor	Post-transplant CMV infection	Tablet	Y	Y
TG Therapeutics	Ukoniq	Umbralisib	PI3K δ and CK1 ϵ inhibitor	MZL, follicular lymphoma	Tablet		Y
ViiV	Cabenuva	Cabotegravir; rilpivirine	INSTI and an NNRTI	HIV-1 infection	Injection		
Zealand Pharma	Zegalogue	Dasiglucagon	Glucagon receptor agonist	Severe hypoglycaemia	Injection		

Sponsor	Biologic	Properties	Indication
Cell/Gene Therapy			
BMS/Celgene	Idecabtagene vicleucel (Abecma)	BCMA-targeted CAR-T cell therapy	Multiple myeloma
BMS/Juno	Lisocabtagene maraleucel (Breyanzi)	CD19-targeted CAR-T cell therapy	DLBCL (diffuse large B-cell lymphoma)
Enzyvant	Rethymic	Allogeneic processed thymus tissue	Congenital athymia
Vaccine			
Merck & Co.	Vaxneuvance	Pneumococcal 15-valent conjugate vaccine	Streptococcus pneumoniae prevention
Pfizer/BioNTech	Comirnaty	COVID-19 mRNA vaccine	COVID-19 prevention
Pfizer	Pevnar 20	Pneumococcal 20-valent conjugate vaccine	Streptococcus pneumoniae prevention
Pfizer	TicoVac	Tick-borne encephalitis vaccine	Tick-borne encephalitis prevention

- COVID-19 관련 프로젝트들 중, full approval을 받은 신약은 Comirnaty (Pfizer/BioNTech)이 유일함

2021년 FDA Emergency Use Authorization

Sponsor	Therapeutics	Properties	Indication
AstraZeneca	Tixagevimab plus cilgavimab	Spike-targeted mAbs	COVID-19 prophylaxis
Eli Lilly	Bamlanivimab plus etesevimab	Spike-targeted mAbs	Mild-to-moderate COVID-19
GlaxoSmithKline	Sotrovimab	Spike-targeted mAb	Mild-to-moderate COVID-19
J&J	JNJ-78436735	Viral vector vaccine	COVID-19 prevention
Merck & Co.	Molnupiravir	Nucleoside analogue	Mild-to-moderate COVID-19
Pfizer	Nirmatrelvir plus ritonavir	MPRO inhibitor plus cytochrome p450 inhibitor	Mild-to-moderate COVID-19

- 2021년 Emergency Use가 허가된 신약은 모두 COVID-19 치료제/백신이었습니다

2021년 FDA 승인 신약 중 블록버스터 예상 신약

Sponsor	Trade name	Properties	Indication	2026 US sales
Novartis/Alnylam	Leqvio	PCSK9-targeted siRNA	HeFH or ASCVD	\$1.7bn
BMS/Celgene	Abecma	BCMA-targeted CAR-T cell therapy	Multiple myeloma	\$1.4bn
Pfizer	Cominaty	COVID-19 mRNA vaccine	COVID-19 prevention	\$1.4bn
AstraZeneca/Amgen	Tezspire	TSLP-targeted mAb	Severe asthma	\$1.2bn
Argenx	Vyvgart	FcRn-binding Fc fragment	Myasthenia gravis	\$1.2bn
Amgen	Lumakras	KRAS-G12C inhibitor	KRASG12C-mutated NSCLC	\$1.2bn
Aurinia	Lupkynis	Calcineurin inhibitor	Lupus nephritis	\$1.1bn

- Aduhelm(Biogen)은 가장 주목 받는 신약이었으나, 미국 보건복지부 산하 메디케어 메디케이드 서비스센터가 보험적용을 임상에 참여하는 환자로 제한함에 따라, 대부분 환자의 보험적용이 어려워지며 블록버스터 예상 신약에서 제외됨 (1/11). 최종 급여결정 여부는 4/11에 확정될 예정

2022년 승인 예상 주요신약

Sponsor	Drug	Properties	Indication	Expected PDUFA
Roche	Faricimab	Angiopoietin-2×VEGF-A bispecific	nAMD and DME	Jan
Sanofi	Sutimlimab	C1s-targeted mAb	AIHA	Feb
Immunocore	Tebentafusp	gp100-targeted TCR fused to CD3-binding Fab	Uveal melanoma	Feb
Gilead	Lenacapavir	Viral capsid inhibitor	HIV	Feb
J&J	Ciltacabtagene autoleucel	BCMA-targeted CAR-T cell therapy	Multiple myeloma	Feb
BMS	Relatlimab plus nivolumab	LAG3-targeted mAb plus PD1 targeted mAb	Melanoma	March
Anylam	Vutrisiran	Transthyretin-targeted siRNA	hATTR	April
Moderna	Elasomeran	mRNA vaccine	COVID-19 prevention	April
BMS	Mavacamten	Myosin inhibitor	Cardiomyopathy	April
Bluebird Bio	Betibeglogene autotemcel	Lentiviral beta-globin gene therapy	β-thalassemia	May
Eli Lilly	Tirzepatide	GIP/GLP1 co-agonist peptide	Type 2 diabetes	May
Novartis/Endocyte	Lutetium 177Lu-PSMA-617	PSMA-targeted radiopharmaceutical	Prostate cancer	First half 2022
BMS	BMS-986165	TYK2 inhibitor	Psoriasis	Sept
Novavax	NVX-CoV2373	Protein subunit vaccine	COVID-19 prevention	EUA in 2022
Eli Lilly	Donanemab	Amyloid-β-targeted mAb	Alzheimer's disease	Filing by Q1
Biogen/Eisai	Lecanemab	Amyloid-β-targeted mAb	Alzheimer's disease	Filing by Q2
Mirati	Adagrasib	KRAS-G12C inhibitor	NSCLC	Filing planned
Pfizer	Nirmatrelvir plus ritonavir	MPRO inhibitor plus cytochrome P450 inhibitor	COVID-19 infection	Filing planned

- FDA, Novel Drug Approvals for 2021, 2022.1
- Nature Review Drug Discovery, 2021 FDA approvals, 2022.1
- Evaluate Vantage, Evaluate Vantage 2022 Preview, 2021.12