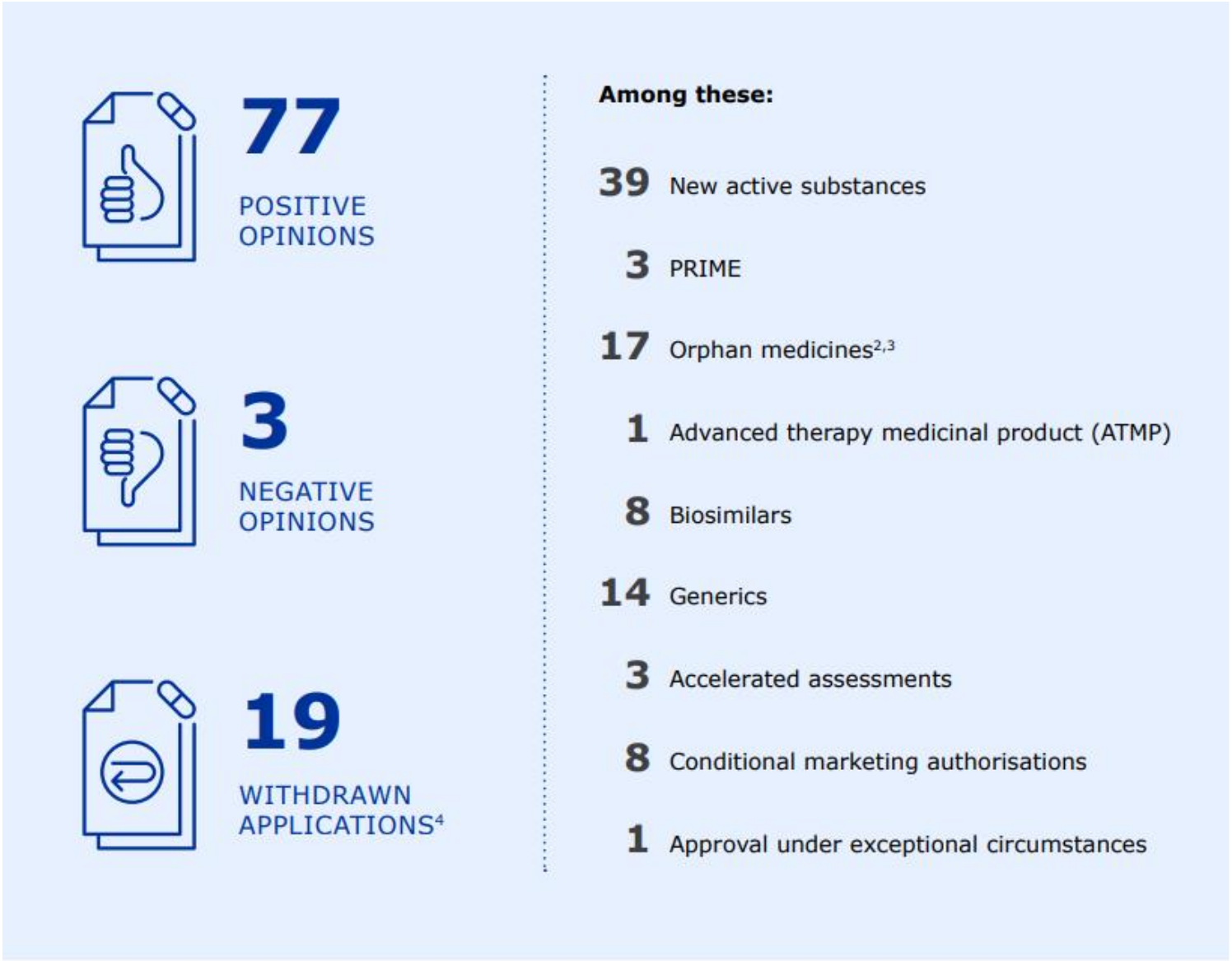
2023년 EMA 신약 승인 현황

국가신약개발사업단 기획팀 진주연 연구원

OVERVIEW

지난 1월 유럽 EMA는 'Human Medicines Highlights 2023'을 통해 지난 2023년 한 해 동안 진행한 심사 결과를 공개했다. 2023년 EMA가 판매 승인을 권고하는 '긍정적인 의견(Positive opinions)'을 받은 의약품은 총 77건이며, '부정적인 의견(Negative opinions)'을 받은 의약품은 3건, '반려된 의약품(Withdrawn applications)'은 19건이다.

긍정적인 의견을 받은 의약품(77개) 중에는 새로운 유효 성분(New active substances)이 39건, PRIME(Priority Medicines) 3건, 희귀의약품(Orphan medicines) 17건, 첨단치료 의약품(Advanced therapy medicinal products) 1건, 바이오시밀러(Biosimilars) 8건, 제네릭(Generics) 14건, 신속심사(Accelerated assessments) 3건, 조건부허가(Conditional marketing authorisations) 8건, 예외적 상황의 품목허가(Approvals under exceptional circumstances) 1건이 포함된다(그림 1).



[그림 1] 2023 EMA 심사 결과[1)]

1) 그림 1은 EU 전역의 판매 허가를 위한 유럽위원회의 EMA 권고 사항을 반영함

2) 이 수치는 2023년 12월 31일까지 희귀의약품 지정이 확정된 의약품을 의미함

3) 판매 허가 보유자의 요청에 따라 1건의 의약품(Tevimbra)이 희귀의약품 지정에서 제외됨

4) 더 자세한 정보는 EMA 웹사이트를 참조

IMPORTANT CONTRIBUTIONS TO PUBLIC HEALTH

아래 표에서는 2023년에 승인된 공중보건에 기여한 주요 의약품 18개를 소개한다(표 1).

질환별로 암 10건(Columvi, Tepkinly, Elrexfio, Finlee, Jaypirca, Krazati, Lytgobi, Omjjara, Pedmarqsi, Talvey), 심혈관계 2건(Aqumeldi, Camzyos), 혈액학 1건(Casgevy), 대사 1건(Loargys), 신경계 1건(Skyclarys), 백신 3건(Abrysvo, Arexvy, Bimervax)이 있다.

<표 1> 18 important contributionbs to public health

No.	적응증	제품명	성분명	모달리티	회사명
CANCER					
1	Diffuse large B-cell lymphoma	Columvi	Glofitamab	CD20 × CD3 T-cell engager	Genentech
2	Diffuse large B-cell lymphoma	Tepkinly	Epcoritamab	CD20 × CD3 T-cell engager	Genmab/AbbVie
3	Relapsed or refractory multiple myeloma	Elrexfio	Elranatamab	BCMA × CD3 T-cell engager	Pfizer
4	Paediatric patients aged one year and older with glioma	Finlee+ (Spexotras 병용)	dabrafenib+ (trametinib 병용)	BRAF kinases inhibitor	Novartis
5	Relapsed or refractory mantle cell lymphoma	Jaypirca	Pirtobrutinib	BTK inhibitor	Loxo/Eli Lilly
6	Non-small cell lung cancer	Krazati	Adagrasib	KRAS-G12C inhibitor	Mirati
7	Cholangiocarcinoma or bile duct cancer	Lytgobi	Futibatinib	FGFR kinase inhibitor	Taiho Oncology
8	Myelofibrosis	Omjjara	Momelotinib	JAK1/2, ALK2 inhibitor	GSK
9	Prevention of ototoxicity	Pedmarqsi	Sodium thiosulfate	Cisplatin neutralizing agent	Fennec Pharmaceuticals
10	Relapsed and refractory multiple myeloma	Talvey	Talquetamab	GPRC5D × CD3 T-cell engager	Janssen
CARDIOVASCULAR					

No.	적응증	제품명	성분명	모달리티	회사명
11	Heart failure in children from birth to less than 18 years	Aqumeldi	Enalapril maleate	Angiotensin-converting enzyme (ACE) inhibitor	Proveca Pharma
12	Symptomatic obstructive hypertrophic cardiomyopathy	Camzyos	Mavacamten	Myosin 7 inhibitor	Bristol-Myers Squibb
HAEMATOLOGY					
13	Transfusion-dependent beta-thalassemia and severe sickle cell disease	Casgevy	Exagamglogene autotemcel	CRISPR–Cas9-based BCL11a-gene-editing therapy	Vertex/CRISPR
METABOLISM					
14	Hyperargininaemia	Loargys	Pegzilarginase	Recombinant human arginase 1 enzyme	Spyre Therapeutics
NEUROLOGY					
15	Friedreich's ataxia	Skyclarys	Omaveloxolone	NRF2 activator	Reata/Biogen
VACCINES					
16	Respiratory syncytial virus (RSV)	Abrysvo	Subunit vaccine, recombinant	Bivalent stabilized prefusion F subunit vaccine	Pfizer
17	Respiratory syncytial virus (RSV)	Arexvy	Subunit vaccine, recombinant	Stabilized prefusion F subunit vaccine	GSK
18	COVID-19	Bimervax	Subunit vaccine, recombinant	Recombinant Protein RBD Candidate Vaccine	HIPRA

EARLY ACCESS TO MEDICINES THAT ADDRESS PUBLIC HEALTH NEEDS

1. ACCELERATED ASSESSMENTS

2023년 EMA의 가속승인을 받은 의약품은 3건으로 암 치료제 1건(Talvey), RSV 백신 2건(Abrysvo, Arexvy)이다.

<표 2> 3 accelerated assessments

No.	적응증	제품명	성분명	모달리티	회사명
CANCER					
1	Relapsed and refractory multiple myeloma	Talvey	Talquetamab	GPRC5D × CD3 T-cell engager	Janssen
VACCINES					
2	Respiratory syncytial virus (RSV)	Abrysvo	Subunit vaccine, recombinant	Bivalent stabilized prefusion F subunit vaccine	Pfizer
3	Respiratory syncytial virus (RSV)	Arexvy	Subunit vaccine, recombinant	Stabilized prefusion F subunit vaccine	GSK

2. PRIORITY MEDICINES(PRIME)

우선심사 지정(PRIME)을 통해 승인 받은 의약품은 3건으로 암 치료제 2건(Elrexfio, Talvey)과 최초의 CRISPR 기술 기반 치료제인 Casgevy를 포함한다.

<표 3> 3 priority medicines

No.	적응증	제품명	성분명	모달리티	회사명
CANCER					

No.	적응증	제품명	성분명	모달리티	회사명
1	Relapsed or refractory multiple myeloma	Elrexfio	Elranatamab	BCMA × CD3 T-cell engager	Pfizer
2	Relapsed and refractory multiple myeloma	Talvey	Talquetamab	GPRC5D × CD3 T-cell engager	Janssen
HAEMATOLOGY					
3	Transfusion-dependent beta-thalassemia and severe sickle cell disease	Casgevy	Exagamglogene autotemcel	CRISPR–Cas9-based BCL11a-gene-editing therapy	Vertex/CRISPR

3. MEDICINES FOR RARE DISEASES

희귀 의약품은 총 17건으로 질환별로는 암 8건(Columvi, Finlee, Omjjara, Spexotras, Talvey, Tepkinly, Tevimbra, Tibsovo), 피부과 1건(Hyftor), 내분비학 1건(Yorvipath), 혈액학 1건(Casgevy), 감염 1건(Rezzayo), 대사 1건(Loargys), 신경학 4건(Agamree, Rystiggo, Skyclarys, Ztalmy)이 있다.

<표 4> 17 medicines for rare diseases

No.	적응증	제품명	성분명	모달리티	회사명
CANCER					
1	Diffuse large B-cell lymphoma	Columvi	Glofitamab	CD20 × CD3 T-cell engager	Genentech
2	Paediatric patients aged one year and older with glioma	Finlee	Dabrafenib	BRAF kinases inhibitor	Novartis
3	Myelofibrosis	Omjjara	Momelotinib	JAK1/2, ALK2 inhibitor	GSK
4	Low-grade glioma, high-grade glioma	Spexotras	Trametinib	MEK inhibitor	Novartis
5	Relapsed and refractory multiple myeloma	Talvey	Talquetamab	GPRC5D × CD3 T-cell engager	Janssen
6	Diffuse large B-cell lymphoma	Tepkinly	Epcoritamab	CD20 × CD3 T-cell engager	Genmab/AbbVie
7	Squamous oesophageal cancer	Tevimbra*	Tislelizumab	PD1 antagonist	BeiGene

No.	적응증	제품명	성분명	모달리티	회사명
8	Acute myeloid leukaemia	Tibsovo	Ivosidenib	IDH1(isocitrate dehydrogenase 1) enzyme inhibitor	Servier
Dermatology					
9	Facial angiofibroma	Hyftor	Sirolimus	mTOR inhibitor	Nobel pharma
Endocrinology					
10	Chronic hypoparathyroidism	Yorvipath	Palopegteriparatide	PTH(parathyroid hormone) replacement therapy	Ascendis Pharma
HAEMATOLOGY					
11	Transfusion-dependent beta-thalassemia and severe sickle cell disease	Casgevy	Exagamglogene autotemcel	CRISPR–Cas9-based BCL11a-gene-editing therapy	Vertex/CRISPR
INFECTIONS					
12	Fungal infection	Rezzayo	Rezafungin	echinocandin antifungal agent	Cidara Therapeutics
METABOLISM					
13	Hyperargininaemia	Loargys	Pegzilarginase	Recombinant human arginase 1 enzyme	Spyre Therapeutics
NEUROLOGY					
14	Duchenne muscular dystrophy in patients from 4 years of age	Agamree	Vamorolone	Corticosteroid	Santhera Pharmaceuticals
15	Generalised myasthenia gravis	Rystiggo	Rozanolixizumab	FcRn-targeted mAb	UCB
16	Friedreich's ataxia	Skyclarys	Omaveloxolone	Mechanism unknown, NRF2 activator	Reata/Biogen
17	Cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder	Ztalmy	Ganaxolone	GABAA receptor positive allosteric modulator	Marinus Pharmaceuticals

※ Tevimbra: 판매 허가 보유자의 요청에 따라 희귀의약품 지정에서 제외됨

NEGATIVE OPINIONS

부정적인 의견을 받은 의약품은 3건으로 Albrioza, Lagevrio, Sohonos가 있다.

<표 5> 3 Negative opinions

No.	적응증	제품명	성분명	모달리티	회사명
1	Amyotrophic lateral sclerosis	Albrioza	Sodium phenylbutyrate/ ursodoxicoltaurine	Dual UPR(unfolded protein response)-Bax apoptosis inhibitor	Amylyx Pharmaceuticals
2	COVID-19 treatment	Lagevrio	Molnupiravir	RNA Directed RNA Polymerase inhibitor	Merk
3	Fibrodysplasia ossificans progressiva	Sohonos	Palovarotene	Retinoic acid receptor agonist	Ipsen

[참고] MEDICINES RECOMMENDED FOR APPROVAL[4]

THERAPEUTIC AREA/ PRODUCT NAME	New active substance	PRIME	Orphan	ATMP	Biosimilar	Generic	Accelerated assesment	Conditional approval	Exceptional circumstances
Cancer									
Akeega									
Azacitidine Kabi						●			
Columvi	●		●					●	
Degarelix Accord						●			
Elrexfio	●	●						●	
Enrylaze									
Finlee			●						
Herwenda					●				
Inaqovi	●								
Jaypirca	●							●	
Krazati	●							●	
Lytgobi	●							●	
Mevlyq						●			
Naveruclif						●			
Omjjara	●		●						
Orserdu	●								
Pedmarqsi									
Pomalidomide Viatris						●			
Spexotras			●						
Talvey	●	●	●				●	●	
Tepkinly	●		●					●	
Tevimbra[5]	●		●						
Tibsovo	●		●						
Tidhesco[6]									
Vanflyta	●								

[4] Some medicines might fall into more than one therapeutic area but have been reflected only in one.
[5] The orphan status was removed after authorisation at the request of the marketing authorisation holder.
[6] Duplicate of Tibsovo. The marketing authorisation application was withdrawn after the positive CHMP opinion.

THERAPEUTIC AREA/ PRODUCT NAME	New active substance	PRIME	Orphan	ATMP	Biosimilar	Generic	Accelerated assesment	Conditional approval	Exceptional circumstances
Cardiovascular									
Aqumeldi									
Camzyos	●								
Dabigatran Etexilate Accord						●			
Dabigatran Etexilate Leon Farma						●			
Ibuprofen Gen.Orph						●			
Qaialdo									
Dermatology									
Ebglyss	●								
Hyftor			●						
Opzelura									
Litfulo	●								
Sotyktu	●								
Diagnostic agents									
Elucirem	●								
Pylclari	●								
Vueway[7]									
Endocrinology									
Dapagliflozin Viatris						●			
Sitagliptin/Metformin hydrochloride Sun						●			
Tolvaptan Accord						●			
Yorvipath	●		●						
Veoza	●								
Gastroenterology/ Hepatology									
Omvoh	●								
Velsipity	●								
Haematology/ Haemostaseology									
Bekemv					●				
Casgevy	●	●	●	●				●	
Epysqli					●				
Jesduvroq	●								
Vafseo	●								

[7] Duplicate of Elucirem

THERAPEUTIC AREA/ PRODUCT NAME	New active substance	PRIME	Orphan	ATMP	Biosimilar	Generic	Accelerated assesment	Conditional approval	Exceptional circumstances
Immunology/ Rheumatology/ Transplantation									
Tyenne					●				
Uzpruvo					●				
Infections									
Apretude									
Rezzayo	●		●						
Metabolism									
Elfabrio									
Loargys	●		●						●
Opfolda									
Neurology									
Agamree	●		●						
Aquipta	●								
Briumvi	●								
Lacosamide Adroiq						●			
Rystiggo	●		●						
Skyclarys	●		●						
Sugammadex Adroiq						●			
Sugammadex Piramal						●			
Tyruko					●				
Zilbrysq	●								
Ztalmy	●		●						
Ophthalmology									
Catiolanze									
Rimmyrah					●				
Yesafili					●				
Pneumology/Allergology									
Lyfnua	●								
Vaccines									
Abrysvo	●						●		
Arexvy	●						●		
Bimervax	●								
Zoonotic Influenza Vaccine Seqirus									

<문의>

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Reference

EMA, Human Medicines Highlights 2023

https://www.ema.europa.eu/