

Supplemental Information: Additional file 2 – The research material

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EMA approved orphan medicines since the implementation of the orphan legislation, Orphanet Journal of Rare Diseases

Eveliina Hahl ^{1,*}, Terhi Kurko ², Hanna Koskinen ², Marja Airaksinen ¹ and Kati Sarnola ²

* Correspondence: eveliina.hahl@helsinki.fi

¹ Faculty of Pharmacy, University of Helsinki, Helsinki, Finland

² Research Unit, The Social Insurance Institution of Finland, Helsinki, Finland

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Tradename ¹	Active substance	Reimbursable	Outpatient medicine ²	Medicine used in inpatient care ²
Adcetris	brentuximab vedotin	no		x
Adempas	riociguat	yes	x	
Alofisel	darvadstrocel	no	x	
Alprolix	eftrenonacog alfa	yes	x	
Arikayce liposomal	amikacin	no	x	
Aspaveli	pegcetacoplan	yes		x
Besponsa	inotuzumab ozogamicin	no		x
Blinicyto	blinatumomab	no		x
Cablivi	caplacizumab	no	x	
Cerdelga	eliglustat	yes	x	
Cometriq	cabozantinib	yes	x	
Cresemba	isavuconazole	yes	x	

Tradename ¹	Active substance	Reimbursable	Outpatient medicine ²	Medicine used in inpatient care ²
Crysvita	burosumab	yes	x	
Cystadrops	mercaptopamine	no	x	
Dacogen	decitabine	no		x
Darzalex	daratumumab	yes	x	
Defitelio	defibrotide	no		x
Enspryng	satralizumab	no	x	
Epidyolex	cannabidiol	yes	x	
Evrysdi	risdiplam	yes	x	
Fintepla	fenfluramine	yes	x	
Galafold	migalastat	yes	x	
Gazyvaro	obinutuzumab	no		x
Hepcludex	bulevirtide	no	x	
Iclusig	ponatinib	yes	x	
Idefirix	imlifidase	no		x
Imnovid (Pomalidomide Celgene)	pomalidomide	yes	x	
Isturisa	osilodrostat	no	x	
Jorveza	budesonide	yes	x	
Kaftrio	ivacaftor / tezacaftor / elexacaftor	yes	x	
Kalydeco	ivacaftor	yes	x	
Kanuma	sebelipase alfa	no		x
Ketoconazole HRA	ketoconazole	yes	x	
Kimmtrak	tebentafusp	no		x
Koselugo	selumetinib	no	x	
Kymriah	tisagenlecleucel	no		x
Kyprolis	carfilzomib	no		x
Ledaga	chlormethine	yes	x	
Minjuvi	tafasitamab	no		x

Tradename ¹	Active substance	Reimbursable	Outpatient medicine ²	Medicine used in inpatient care ²
Mylotarg	gemtuzumab ozogamicin	no		x
Natpar	parathyroid hormone	no	x	
Nexavar	sorafenib	yes	x	
NexoBrid	Concentrate of proteolytic enzymes enriched in bromelain	no	x	
Ngenla	somatogon	yes	x	
Ninlaro	ixazomib	yes	x	
Ocaliva	obeticholic acid	yes	x	
Onivyde pegylated liposomal	irinotecan	no		x
Opsumit	macitentan	yes	x	
Oxbryta	voxelotor	no	x	
Pemazyre	pemigatinib	yes	x	
Polivy	polatuzumab vedotin	no		x
Poteligeo	mogamulizumab	no		x
Prevymis	letermovir	yes	x	
Procysbi	mercaptamine (cysteamine bitartrate)	no	x	
Qarziba	dinutuximab beta	no		x
Ravicti	glycerol phenylbutyrate	yes	x	
Raxone	idebenone	yes	x	
Reblozyl	luspatercept	no	x	
Revestive	teduglutide	yes	x	
Rydapt	midostaurin	yes	x	
Signifor	pasireotide	yes	x	
Sirturo	bedaquiline	no	x	
Soliris	eculizumab	no		x
Spinraza	nusinersen	no	x	
Strensiq	asfotase alfa	yes	x	

Tradename ¹	Active substance	Reimbursable	Outpatient medicine ²	Medicine used in inpatient care ²
Sylvant	siltuximab	no		x
Symkevi	tezacaftor/ivacaftor	yes	x	
Takhzyro	lanadelumab	yes	x	
Tavneos	avacopan	yes	x	
Tecartus	brexucabtagene autoleucel	no		x
Tobi Podhaler	tobramycin	yes	x	
Translarna	ataluren	yes	x	
Trecondi	treosulfan	no		x
Voraxaze	glucarpidase	no	x	
Votubia	everolimus	yes	x	
Vpriv	velaglucerase alfa	yes		x
Vyndaqel	tafamidis	yes	x	
Vyxeos liposomal	daunorubicin / cytarabine	no		x
Wakix	pitolisant hydrochloride	yes	x	
Xermelo	telotristat	no	x	
Xospata	gilteritinib	yes	x	
Yescarta	axicabtagene ciloleucel	no		x
Zejula	niraparib	yes	x	
Zolgensma	onasemnogene abeparvovec	no		x

¹Every novel orphan medicine is only one time in the table. Not including extensions of indications.

²Classified by dosage form. Infusions are classified as medicine used in inpatient care; other medicines are classified as outpatient medicines.

Table S4 Orphan medicinal products on the market in Finland in May 2024 which had expired orphan designation in May 2022

Tradename ¹	Active substance	Reimbursable	Outpatient medicine ²	Medicine used in inpatient care ²
Afinitor	everolimus	yes	x	
Aldurazyme	laronidase	no		x
Atriance	nelarabine	no		x
Bavencio	avelumab	no		x
Bosulif	bosutinib	yes	x	
Carbaglu	carglumic acid	no	x	
Cyramza	ramucirumab	no		x
Cystadane	betaine anhydrous	yes	x	
Diacomit	stiripentol	yes	x	
Elaprase	Idursulfase	no		x
Esbriet	pirfenidone	no	x	
Exjade	deferasirox	yes	x	
Fabrazyme	agalsidase beta	yes		x
Firazyr	icatibant	yes	x	
Firdapse	amifampridine	no	x	
Gliolan	5-aminolevulinic acid hydrochloride	no	x	
Glivec	imatinib	yes	x	
Ilaris	canakinumab	no	x	
Imbruvica	ibrutinib	yes	x	
Inovelon	rufinamide	yes	x	
Jakavi	ruxolitinib	yes	x	
Lenvima	lenvatinib	yes	x	
Litak	cladribine	no	x	
Lynparza	olaparib	yes	x	
Lysodren	mitotane	yes	x	
Mozobil	plerixafor	no	x	

Tradename ¹	Active substance	Reimbursable	Outpatient medicine ²	Medicine used in inpatient care ²
Myozyme	alglucosidase alfa	no		x
Nexavar	sorafenib	yes	x	
Nplate	romiplostim	yes	x	
Ofev	nintedanib	yes	x	
Orfadin	nitisinone	yes	x	
Pedea	ibuprofen	no	x	
Peyona	caffeine citrate	no	x	
Prialt	ziconotide	no		x
Replagal	agalsidase alfa	yes		x
Revatio	sildenafil	yes	x	
Revlimid	lenalidomide	no	x	
Revolade	eltrombopag	yes	x	
Savene	dexrazoxane	no		x
Signifor	pasireotide	yes	x	
Siklos	hydroxycarbamide	no	x	
Soliris	eculizumab	no		x
Somavert	pegvisomant	yes	x	
Sprycel	dasatinib	no	x	
Sutent	sunitinib	yes	x	
Tasigna	nilotinib	yes	x	
Tepadina	thiotepa	no		x
Thalidomide BMS	thalidomide	no	x	
Torisel	temsirolimus	no		x
Tracleer	bosentan	yes	x	
Trisenox	arsenic trioxide	no		x
Venclyxto	venetoclax	yes	x	
Ventavis	iloprost	yes	x	

Tradename ¹	Active substance	Reimbursable	Outpatient medicine ²	Medicine used in inpatient care ²
Vidaza	azacitidine	no	x	
Volibris	ambrisentan	yes	x	
Vyndaqel	tafamidis	yes	x	
Wilzin	zinc acetate dihydrate	no	x	
Xagrid	anagrelide	yes	x	
Xaluprine	mercaptopurine	yes	x	
Xyrem	sodium oxybate	yes	x	
Yondelis	trabectedin	no		x
Zavesca	miglustat	yes	x	

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