

Lista e produkteve orfane

29.02.2024

EU #	Product	Indication	Sponsor	Designation date	Tradename - EU product # - Implemented on
EU/3/17/1863	Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains	Treatment of multiple myeloma	Bristol-Myers Squibb Pharma EEIG	20 Apr 2017	Abecma - EU/1/21/1539 - 19 Aug 2021
EU/3/11/939	Brentuximab vedotin	Treatment of cutaneous T-cell lymphoma	Takeda Pharma A/S	11 Jan 2012	ADCETRIS - EU/1/12/794 - 19 Dec 2017
EU/3/08/596	Monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E	Treatment of Hodgkin lymphoma	Takeda Pharma A/S	15 Jan 2009	ADCETRIS - EU/1/12/794 - 30 Oct 2012
EU/3/08/595	Monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E	Treatment of peripheral T-cell lymphoma	Takeda Pharma A/S	15 Jan 2009	ADCETRIS - EU/1/12/794 - 30 Oct 2012
EU/3/14/1309	17 α ,21-dihydroxy-16 α -methyl-pregna-1,4,9(11)-triene 3,20-dione	Treatment of Duchenne muscular dystrophy	Santhera Pharmaceuticals (Deutschland) GmbH	22 Aug 2014	AGAMREE - EU/1/23/1776 - 15 Dec 2023
EU/3/09/667	Expanded human allogeneic mesenchymal adult stem cells extracted from adipose tissue	Treatment of anal fistula	Takeda Pharma A/S	08 Oct 2009	Alofisel - EU/1/17/1261 - 27 Mar 2018
EU/3/07/453	Recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1	Treatment of haemophilia B (congenital factor IX deficiency)	Swedish Orphan Biovitrum AB (publ)	08 Jun 2007	ALPROLIX - EU/1/16/1098 - 13 May 2016
EU/3/15/1589	Glibenclamide	Treatment of neonatal diabetes	AMMTeK	15 Jan 2016	AMGLIDIA - EU/1/18/1279 - 28 May 2018

EU/3/18/2026	Synthetic double-stranded siRNA oligonucleotide targeted against transthyretin mRNA, with six phosphorothioate linkages in the backbone, and nine 2'-fluoro and thirty-five 2'-O-methyl nucleoside residues in the sequence, which is covalently linked via a phosphodiester group to a ligand containing three N-acetylgalactosamine residues	Treatment of transthyretin-mediated amyloidosis	Alnylam Netherlands B.V.	25 May 2018	Amvuttra - EU/1/22/1681 - 16 Sep 2022
EU/3/14/1259	Amikacin sulfate	Treatment of nontuberculous mycobacterial lung disease	Insmed Netherlands B.V.	08 Apr 2014	ARIKAYCE liposomal - EU/1/20/1469 - 28 Oct 2020
EU/3/20/2251	Artesunate	Treatment of malaria	Amivas Ireland Limited	28 Feb 2020	Artesunate Amivas - EU/1/21/1582 - 24 Nov 2021
EU/3/17/1873	Poly(oxy-1,2-ethanediyl), .alpha.-hydro-.omega.-hydroxy-,15,15'-diester with N-acetyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminy-L-.alpha.-aspartyl-L-tryptophylglycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-L-threonyl-2-[2-(2-aminoethoxy)ethoxy]acetyl-N6-carboxy-L-lysine cyclic (2.fwdarw.12)-(disulfide); where two identical synthetic peptide domains are covalently linked at the ends of the polyethylene glycol chain	Treatment of paroxysmal nocturnal haemoglobinuria	Swedish Orphan Biovitrum AB (publ)	22 May 2017	ASPAVELI - EU/1/21/1595 - 14 Dec 2021
EU/3/18/2074	Avapritinib	Treatment of mastocytosis	Blueprint Medicines (Netherlands) B.V.	26 Oct 2018	AYVAKYT - EU/1/20/1473 - 25 Mar 2022
EU/3/17/1889	(S)-1-(4-fluorophenyl)-1-(2-(4-(6-(1-methyl-1H-pyrazol-4-yl)pyrrolo[2,1-f][1,2,4]triazin-4-yl)piperazin-yl)pyrimidin-5-yl)ethan-1-amine	Treatment of gastrointestinal stromal tumours	Blueprint Medicines (Netherlands) B.V.	17 Jul 2017	AYVAKYT - EU/1/20/1473 - 25 Sep 2020
EU/3/17/1925	Humanised monoclonal antibody targeting B-cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F	Treatment of multiple myeloma	GlaxoSmithKline (Ireland) Limited	16 Oct 2017	BLENREP - EU/1/20/1474 - 26 Aug 2020
EU/3/09/650	Blinatumomab	Treatment of acute lymphoblastic leukaemia	Amgen Europe B.V.	24 Jul 2009	BLINCYTO - EU/1/15/1047 - 25 Nov 2015

EU/3/13/1118	Recombinant human tripeptidyl-peptidase 1	Treatment of neuronal ceroid lipofuscinosis type 2	BioMarin International Limited	12 Mar 2013	Brineura - EU/1/17/1192 - 01 Jun 2017
EU/3/12/1028	(2S)-2-(((2R)-2-(((3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro-1,2,5-benzothiadiazepin-8-yl)oxy)acetyl)amino)-2-(4-hydroxyphenyl)acetyl]amino)butanoic acid	Treatment of progressive familial intrahepatic cholestasis	Albireo AB	17 Jul 2012	Bylvay - EU/1/21/1566 - 19 Jul 2021
EU/3/09/629	Nanobody directed towards the human A1 domain of von Willebrand factor	Treatment of thrombotic thrombocytopenic purpura	Ablynx N.V.	30 Apr 2009	Cablivi - EU/1/18/1305 - 04 Sep 2018
EU/3/20/2252	Autologous human T cells genetically modified ex-vivo with a lentiviral vector encoding a chimeric antigen receptor for B-cell maturation antigen	Treatment of multiple myeloma	Janssen-Cilag International NV	28 Feb 2020	CARVYKI - EU/1/22/1648 - 30 May 2022
EU/3/19/2242	Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene	Treatment of sickle cell disease	Vertex Pharmaceuticals (Ireland) Limited	09 Jan 2020	Casgevy - EU/1/23/1787 - 12 Feb 2024
EU/3/19/2210	Autologous CD34+ haematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene	Treatment of beta-thalassaemia intermedia and major	Vertex Pharmaceuticals (Ireland) Limited	17 Oct 2019	Casgevy - EU/1/23/1787 - 12 Feb 2024
EU/3/07/514	(1R, 2R)-Octanoic acid [2-(2',3'-dihydro-benzo [1,4] dioxin-6'-yl)-2-hydroxy-1-pyrrolidin-1-ylmethyl-ethyl]-amide-L-tartaric acid salt	Treatment of Gaucher Disease	Sanofi B.V.	04 Dec 2007	Cerdelga - EU/1/14/974 - 21 Jan 2015
EU/3/14/1406	Chenodeoxycholic acid	Treatment of inborn errors of primary bile acid synthesis	Leadiant GmbH	16 Dec 2014	Chenodeoxycholic acid Leadiant - EU/1/16/1110 - 12 Apr 2017
EU/3/07/471	Human coagulation factor X	Treatment of hereditary factor X deficiency	BPL Bioproducts Laboratory GmbH	17 Sep 2007	Coagadex - EU/1/16/1087 - 18 Mar 2016
EU/3/21/2497	Glofitamab	Treatment of diffuse large B-cell lymphoma	Roche Registration GmbH	15 Oct 2021	Columvi - EU/1/23/1742 - 10 Jul 2023
EU/3/08/610	Cyclopropane-1,1-dicarboxylic acid [4-(6,7-dimethoxyquinolin-4-yloxy)-phenyl]-amide (4-fluoro-phenyl)-amide, (L)-malate salt	Treatment of medullary thyroid carcinoma	Ipsen Pharma	06 Feb 2009	Cometriq - EU/1/13/890 - 26 Mar 2014
EU/3/18/2011	Burosumab	Treatment of phosphaturic mesenchymal tumour	Kyowa Kirin Holdings B.V.	16 Apr 2018	CRYSVITA - EU/1/17/1262 - 28 Jul 2022

EU/3/14/1351	Recombinant human monoclonal IgG1 antibody for fibroblast growth factor 23	Treatment of X-linked hypophosphataemia	Kyowa Kirin Holdings B.V.	15 Oct 2014	CRYSVITA - EU/1/17/1262 - 21 Feb 2018
EU/3/08/578	Cysteamine hydrochloride	Treatment of cystinosis	Recordati Rare Diseases	07 Nov 2008	Cystadrops - EU/1/15/1049 - 23 Jan 2017
EU/3/06/370	Decitabine	Treatment of acute myeloid leukaemia	Janssen-Cilag International NV	08 Jun 2006	Dacogen - EU/1/12/792 - 24 Sep 2012
EU/3/18/2020	Daratumumab	Treatment of AL amyloidosis	Janssen-Cilag International NV	25 May 2018	Darzalex - EU/1/16/1101 - 23 Jun 2021
EU/3/13/1153	Daratumumab	Treatment of plasma cell myeloma	Janssen-Cilag International NV	17 Jul 2013	Darzalex - EU/1/16/1101 - 24 May 2016
EU/3/17/1923	Glasdegib maleate	Treatment of acute myeloid leukaemia	Pfizer Europe MA EEIG	16 Oct 2017	Daurismo - EU/1/20/1451 - 29 Jun 2020
EU/3/07/524	(R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole	Treatment of tuberculosis	Otsuka Novel Products GmbH	01 Feb 2008	Deltyba - EU/1/13/875 - 30 Apr 2014
EU/3/07/513	(S)-2-nitro-6-(4-(trifluoromethoxy)benzyloxy)-6,7-dihydro-5H-imidazo[2,1-b][1,3]oxazine	Treatment of tuberculosis	Mylan IRE Healthcare Ltd	29 Nov 2007	Dovprela - EU/1/20/1437 - 04 Aug 2020
EU/3/16/1627	Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes	Treatment of post-transplant lymphoproliferative disorder	Pierre Fabre Medicament	21 Mar 2016	Ebvallo - EU/1/22/1700 - 16 Dec 2022
EU/3/15/1567	Recombinant human interleukin-3 truncated diphtheria toxin fusion protein	Treatment of blastic plasmacytoid dendritic cell neoplasm	Stemline Therapeutics B.V.	11 Nov 2015	ELZONRIS - EU/1/20/1504 - 13 Jan 2021
EU/3/16/1609	Humanised IgG4 monoclonal antibody against total complement component 1, subcomponent s	Treatment of autoimmune haemolytic anaemia	Sanofi B.V.	17 Feb 2016	Enjaymo - EU/1/22/1687 - 23 Nov 2022
EU/3/16/1680	Humanised anti-IL-6 receptor monoclonal antibody	Treatment of neuromyelitis optica spectrum disorders	Roche Registration GmbH	27 Jun 2016	Enspryng - EU/1/21/1559 - 29 Jun 2021
EU/3/12/1063	Panobinostat	Treatment of multiple myeloma	Pharmaand GmbH	08 Nov 2012	Farydak - EU/1/15/1023 - 01 Sep 2015

EU/3/10/845	Dry extract from birch bark (DER 5-10 : 1), extraction solvent n-heptane 95% (w/w)	Treatment of epidermolysis bullosa	Amryt Pharmaceuticals Designated Activity Company	23 Feb 2011	Filsuvez - EU/1/22/1652 - 23 Jun 2022
EU/3/20/2372	Dabrafenib mesylate	Treatment of glioma	Novartis Europharm Limited	09 Dec 2020	Finlee - EU/1/23/1767 - 16 Nov 2023
EU/3/17/1836	Fenfluramine hydrochloride	Treatment of Lennox-Gastaut syndrome	UCB Pharma	27 Feb 2017	Fintepla - EU/1/20/1491 - 31 Jan 2023
EU/3/13/1219	Fenfluramine hydrochloride	Treatment of Dravet syndrome	UCB Pharma	18 Dec 2013	Fintepla - EU/1/20/1491 - 21 Dec 2020
EU/3/06/368	1-deoxygalactonojirimycin hydrochloride	Treatment of Fabry disease	Amicus Therapeutics Europe Limited	22 May 2006	Galafold - EU/1/15/1082 - 31 May 2016
EU/3/16/1731	Synthetic double-stranded siRNA oligonucleotide directed against delta-aminolevulinic acid synthase 1 mRNA covalently linked to a ligand containing three N-acetylgalactosamine residues	Treatment of acute hepatic porphyria	Alnylam Netherlands B.V.	29 Aug 2016	Givlaari - EU/1/20/1428 - 04 Mar 2020
EU/3/10/826	Para-aminosalicylic acid	Treatment of tuberculosis	Eurocept International B.V.	17 Dec 2010	Granupas - EU/1/13/896 - 09 Apr 2014
EU/3/18/1999	Recombinant adeno-associated viral vector containing a codon-optimized Padua derivative of human coagulation factor IX cDNA	Treatment of haemophilia B	CSL Behring GmbH	21 Mar 2018	Hemgenix - EU/1/22/1715 - 23 Feb 2023
EU/3/15/1500	Synthetic 47-amino-acid N-myristoylated lipopeptide, derived from the preS region of hepatitis B virus	Treatment of hepatitis delta virus infection	Gilead Sciences Ireland Unlimited Company	19 Jun 2015	Hepcludex - EU/1/20/1446 - 05 Aug 2020
EU/3/10/841	Tasimelteon	Treatment of non-24-hour sleep-wake disorders in blind people with no light perception	Vanda Pharmaceuticals Netherlands B.V.	23 Feb 2011	Hetlioz - EU/1/15/1008 - 07 Jul 2015
EU/3/08/579	Ex-vivo expanded autologous human corneal epithelium containing stem cells	Treatment of corneal lesions, with associated corneal (limbal) stem cell deficiency, due to ocular burns	Holostem S.r.l.	07 Nov 2008	Holoclar - EU/1/14/987 - 19 Feb 2015
EU/3/17/1910	Sirolimus	Treatment of tuberous sclerosis	Plusultra Pharma GmbH	23 Aug 2017	Hyftor - EU/1/23/1723 - 26 May 2023

EU/3/16/1826	Recombinant IgG degrading enzyme of Streptococcus pyogenes	Prevention of graft rejection following solid organ transplantation	Hansa Biopharma AB	12 Jan 2017	Idefirix - EU/1/20/1471 - 01 Sep 2020
EU/3/09/723	Recombinant fusion protein linking human coagulation factor IX with human albumin	Treatment of haemophilia B	CSL Behring GmbH	04 Feb 2010	IDELVION - EU/1/16/1095 - 13 May 2016
EU/3/19/2192	Setmelanotide	Treatment of Bardet-Biedl syndrome	Rhythm Pharmaceuticals Netherlands B.V.	21 Aug 2019	Imcivree - EU/1/21/1564 - 05 Sep 2022
EU/3/18/2101	Setmelanotide	Treatment of leptin receptor deficiency	Rhythm Pharmaceuticals Netherlands B.V.	19 Nov 2018	Imcivree - EU/1/21/1564 - 19 Jul 2021
EU/3/16/1703	Setmelanotide	Treatment of pro-opiomelanocortin deficiency	Rhythm Pharmaceuticals Netherlands B.V.	14 Jul 2016	Imcivree - EU/1/21/1564 - 19 Jul 2021
EU/3/10/811	N-tert-butyl-3-[(5-methyl-2-[[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino]pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate	Treatment of post-polycythaemia vera myelofibrosis	Bristol-Myers Squibb Pharma EEIG	26 Nov 2010	Inrebic - EU/1/20/1514 - 09 Feb 2021
EU/3/10/810	N-tert-butyl-3-[(5-methyl-2-[[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino]pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate	Treatment of post-essential thrombocythaemia myelofibrosis	Bristol-Myers Squibb Pharma EEIG	26 Nov 2010	Inrebic - EU/1/20/1514 - 09 Feb 2021
EU/3/10/794	N-tert-butyl-3-[(5-methyl-2-[[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino]pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate	Treatment of primary myelofibrosis	Bristol-Myers Squibb Pharma EEIG	01 Oct 2010	Inrebic - EU/1/20/1514 - 09 Feb 2021
EU/3/14/1345	Osilodrostat	Treatment of Cushing's syndrome	Recordati Rare Diseases	15 Oct 2014	Isturisa - EU/1/19/1407 - 13 Jan 2020
EU/3/13/1181	Budesonide	Treatment of eosinophilic oesophagitis	Dr Falk Pharma GmbH	05 Aug 2013	Jorveza - EU/1/17/1254 - 10 Jan 2018
EU/3/18/2116	Ivacaftor, N-(1,3-dimethyl-1H-pyrazole-4-sulfonyl)-6-[3-(3,3,3-trifluoro-2,2-dimethylpropoxy)-1H-pyrazol-1-yl]-2-[[4S]-2,2,4-trimethylpyrrolidin-1-yl]pyridine-3-carboxamide, tezacaftor	Treatment of cystic fibrosis	Vertex Pharmaceuticals (Ireland) Limited	14 Dec 2018	Kaftrio - EU/1/20/1468 - 21 Aug 2020

EU/3/10/827	Recombinant human lysosomal acid lipase	Treatment of lysosomal acid lipase deficiency	Alexion Europe SAS	17 Dec 2010	Kanuma - EU/1/15/1033 - 01 Sep 2015
EU/3/12/965	Ketoconazole	Treatment of Cushing's syndrome	HRA Pharma Rare Diseases	23 Apr 2012	Ketoconazole HRA - EU/1/14/965 - 21 Nov 2014
EU/3/21/2397	Tebentafusp	Treatment of uveal melanoma	Immunocore Ireland Limited	19 Feb 2021	KIMMTRAK - EU/1/22/1630 - 06 Apr 2022
EU/3/16/1778	Budesonide	Treatment of primary IgA nephropathy	STADA Arzneimittel AG	18 Nov 2016	Kinpeygo - EU/1/22/1657 - 18 Jul 2022
EU/3/18/2050	Selumetinib	Treatment of neurofibromatosis type 1	AstraZeneca AB	31 Jul 2018	Koselugo - EU/1/21/1552 - 19 Jun 2021
EU/3/21/2464	Tisagenlecleucel	Treatment of follicular lymphoma	Novartis Europharm Limited	19 Jul 2021	Kymriah - EU/1/18/1297 - 03 May 2022
EU/3/16/1745	Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19	Treatment of diffuse large B-cell lymphoma	Novartis Europharm Limited	14 Oct 2016	Kymriah - EU/1/18/1297 - 27 Aug 2018
EU/3/14/1266	Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19	Treatment of B-lymphoblastic leukaemia/lymphoma	Novartis Europharm Limited	29 Apr 2014	Kymriah - EU/1/18/1297 - 27 Aug 2018
EU/3/08/548	Carfilzomib	Treatment of multiple myeloma	Amgen Europe B.V.	03 Jun 2008	Kyprolis - EU/1/15/1060 - 23 Nov 2015
EU/3/04/260	Recombinant human α -Mannosidase	Treatment of α -Mannosidosis	Chiesi Farmaceutici S.p.A.	26 Jan 2005	Lamzede - EU/1/17/1258 - 27 Mar 2018
EU/3/12/963	Chlormethine	Treatment of cutaneous T-cell lymphoma	Helsinn Birex Pharmaceuticals Ltd	22 May 2012	Ledaga - EU/1/16/1171 - 07 Mar 2017
EU/3/07/446	Autologous CD34+ cells transfected with lentiviral vector containing the human arylsulfatase A cDNA	Treatment of metachromatic leukodystrophy	Orchard Therapeutics (Netherlands) B.V.	13 Apr 2007	Libmeldy - EU/1/20/1493 - 18 Dec 2020

EU/3/13/1214	(4R,5R)-1-[[4-[[[4-[3,3-dibutyl-7-(dimethylamino)-2,3,4,5-tetrahydro-4-hydroxy-1,1-dioxido-1-benzothiepin-5-yl]phenoxy)methyl]phenyl]methyl]-4-aza-1-azoniabicyclo[2.2.2]octane chloride	Treatment of Alagille syndrome	Mirum Pharmaceuticals International B.V.	18 Dec 2013	Livmarli - EU/1/22/1704 - 12 Dec 2022
EU/3/13/1133	Maribavir	Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity	Takeda Pharmaceuticals International AG Ireland Branch	07 Jun 2013	LIVTENCITY - EU/1/22/1672 - 10 Nov 2022
EU/3/07/519	Maribavir	Prevention of cytomegalovirus (CMV) disease in patients with impaired cell mediated immunity deemed at risk	Takeda Pharmaceuticals International AG Ireland Branch	18 Dec 2007	LIVTENCITY - EU/1/22/1672 - 10 Nov 2022
EU/3/16/1701	Poly(oxy-1,2-ethanediyl), alpha-(carboxymethyl)-omega-methoxy-,amide with arginase 1 [cobalt cofactor] (synthetic human) (1:10), trimer	Treatment of hyperargininaemia	Immedica Pharma AB	14 Jul 2016	Loargys - EU/1/23/1774 - 18 Dec 2023
EU/3/21/2517	Mosunetuzumab	Treatment of follicular lymphoma	Roche Registration GmbH	12 Nov 2021	Lunsumio - EU/1/22/1649 - 07 Jun 2022
EU/3/07/523	Lutetium (177Lu)-N-[(4,7,10-Tricarboxymethyl-1,4,7,10-tetraazacyclododec-1-yl)acetyl]-D-phenylalanyl-L-cysteinyl-L-tyrosyl-D-tryptophanyl-L-lysyl-L-threoninyl-L-cysteinyl-L-threonine-cyclic(2-7)disulfide	Treatment of gastro-entero-pancreatic neuroendocrine tumours	Advanced Accelerator Applications	31 Jan 2008	Lutathera - EU/1/17/1226 - 28 Sep 2017
EU/3/15/1518	Adenovirus-associated viral vector serotype 2 containing the human RPE65 gene	Treatment of inherited retinal dystrophies (initially named treatment of retinitis pigmentosa)	Novartis Europharm Limited	28 Jul 2015	Luxturna - EU/1/18/1331 - 05 Dec 2018
EU/3/12/981	Adenovirus associated viral vector serotype 2 containing the human RPE65 gene	Treatment of inherited retinal dystrophies (initially named treatment of Leber's congenital amaurosis)	Novartis Europharm Limited	02 Apr 2012	Luxturna - EU/1/18/1331 - 05 Dec 2018
EU/3/12/973	Recombinant human beta-glucuronidase	Treatment of mucopolysaccharidosis type VII (Sly syndrome)	Ultragenyx Germany GmbH	21 Mar 2012	Mepsevii - EU/1/18/1301 - 27 Aug 2018
EU/3/14/1424	Humanised Fc engineered monoclonal antibody against CD19	Treatment of diffuse large B-cell lymphoma	Incyte Biosciences Distribution B.V.	15 Jan 2015	Minjuvi - EU/1/21/1570 - 01 Sep 2021

EU/3/12/1025	Metreleptin	Treatment of Berardinelli-Seip syndrome	Amryt Pharmaceuticals Designated Activity Company	17 Jul 2012	Myalepta - EU/1/18/1276 - 01 Aug 2018
EU/3/12/1024	Metreleptin	Treatment of Lawrence syndrome	Amryt Pharmaceuticals Designated Activity Company	17 Jul 2012	Myalepta - EU/1/18/1276 - 01 Aug 2018
EU/3/12/1023	Metreleptin	Treatment of Barraquer-Simons syndrome	Amryt Pharmaceuticals Designated Activity Company	17 Jul 2012	Myalepta - EU/1/18/1276 - 01 Aug 2018
EU/3/12/1022	Metreleptin	Treatment of Familial Partial Lipodystrophy	Amryt Pharmaceuticals Designated Activity Company	17 Jul 2012	Myalepta - EU/1/18/1276 - 01 Aug 2018
EU/3/13/1170	Octreotide acetate (oral use)	Treatment of acromegaly	Amryt Pharmaceuticals Designated Activity Company	05 Aug 2013	Mycapssa - EU/1/22/1690 - 05 Dec 2022
EU/3/00/005	Gemtuzumab Ozogamicin	Treatment of acute myeloid leukaemia	Pfizer Europe MA EEIG	18 Oct 2000	Mylotarg - EU/1/18/1277 - 23 Apr 2018
EU/3/14/1353	Mexiletine hydrochloride	Treatment of myotonic disorders	Lupin Europe GmbH	19 Nov 2014	Namuscla - EU/1/18/1325 - 20 Dec 2018
EU/3/13/1210	Recombinant human parathyroid hormone	Treatment of hypoparathyroidism	Takeda Pharmaceuticals International AG Ireland Branch	18 Dec 2013	Natpar - EU/1/15/1078 - 26 Apr 2017
EU/3/12/1087	Recombinant modified human growth hormone	Treatment of growth hormone deficiency	Pfizer Europe MA EEIG	24 Jan 2013	Ngenla - EU/1/21/1617 - 15 Feb 2022
EU/3/11/899	2,2'-{2-[(1R)-1-([(2,5-dichlorobenzoyl)amino]acetyl)amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid	Treatment of multiple myeloma	Takeda Pharma A/S	27 Sep 2011	Ninlaro - EU/1/16/1094 - 23 Nov 2016
EU/3/10/777	Cyclic pyranopterin monophosphate	Treatment of molybdenum cofactor deficiency type A	TMC Pharma (EU) Limited	20 Sep 2010	NULIBRY - EU/1/22/1684 - 16 Sep 2022
EU/3/18/2065	Obiltoximab	Treatment of anthrax	SFL Pharmaceuticals Deutschland GmbH	24 Aug 2018	NYXTHRACIS - EU/1/20/1485 - 19 Nov 2020

EU/3/10/753	6alpha-ethyl-chenodeoxycholic acid	Treatment of primary biliary cirrhosis	Advanz Pharma Limited	27 Jul 2010	Ocaliva - EU/1/16/1139 - 15 Dec 2016
EU/3/11/888	N-(cyanomethyl)-4-(2-{{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide, dihydrochloride salt	Treatment of primary myelofibrosis	GlaxoSmithKline Trading Services Limited	05 Aug 2011	Omjjara - EU/1/23/1782 - 26 Jan 2024
EU/3/11/887	N-(cyanomethyl)-4-(2-{{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide, dihydrochloride salt	Treatment of post-essential thrombocythaemia myelofibrosis	GlaxoSmithKline Trading Services Limited	05 Aug 2011	Omjjara - EU/1/23/1782 - 26 Jan 2024
EU/3/11/886	N-(cyanomethyl)-4-(2-{{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide, dihydrochloride salt	Treatment of post-polycythaemia vera myelofibrosis	GlaxoSmithKline Trading Services Limited	05 Aug 2011	Omjjara - EU/1/23/1782 - 26 Jan 2024
EU/3/11/933	Nanoliposomal irinotecan	Treatment of pancreatic cancer	Les Laboratoires Servier	09 Dec 2011	Onivyde pegylated liposomal - EU/1/16/1130 - 18 Oct 2016
EU/3/11/857	Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA	Treatment of transthyretin-mediated amyloidosis	Alnylam Netherlands B.V.	15 Apr 2011	Onpattro - EU/1/18/1320 - 29 Aug 2018
EU/3/16/1769	2-hydroxy-6-((2-(1-isopropyl-1H-pyrazol-5-yl)pyridin-3-yl)methoxy)benzaldehyde	Treatment of sickle cell disease	Pfizer Europe MA EEIG	18 Nov 2016	Oxbryta - EU/1/21/1622 - 15 Feb 2022
EU/3/15/1586	Recombinant human nerve growth factor	Treatment of neurotrophic keratitis	Dompé farmaceutici S.p.A.	14 Dec 2015	OXERVATE - EU/1/17/1197 - 10 Jul 2017
EU/3/16/1637	Synthetic double-stranded siRNA oligonucleotide directed against hydroxyacid oxidase 1 mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues	Treatment of primary hyperoxaluria	Alnylam Netherlands B.V.	21 Mar 2016	Oxlumo - EU/1/20/1496 - 23 Nov 2020
EU/3/09/708	Pegylated recombinant phenylalanine ammonia lyase	Treatment of hyperphenylalaninaemia	BioMarin International Limited	28 Jan 2010	Palynziq - EU/1/19/1362 - 08 May 2019
EU/3/18/2066	Pemigatinib	Treatment of biliary tract cancer	Incyte Biosciences Distribution B.V.	24 Aug 2018	Pemazyre - EU/1/21/1535 - 29 Mar 2021
EU/3/16/1756	Mogamulizumab	Treatment of cutaneous T-cell lymphoma	Kyowa Kirin Holdings B.V.	14 Oct 2016	Poteligeo - EU/1/18/1335 - 26 Nov 2018

EU/3/11/849	(S)-{8-fluoro-2-[2-[4-(3-methoxyphenyl)-1-piperazinyl]-3-[2-methoxy-5-(trifluoromethyl)-phenyl]-3,4-dihydro-4-quinazolinyl] acetic acid	Prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk	Merck Sharp & Dohme B.V.	15 Apr 2011	PREVYMIS - EU/1/17/1245 - 10 Jan 2018
EU/3/20/2270	Mitapivat sulfate	Treatment of pyruvate kinase deficiency	Agios Netherlands B.V.	22 Apr 2020	Pyrukynd - EU/1/22/1662 - 10 Nov 2022
EU/3/12/1062	Chimeric monoclonal antibody against GD2	Treatment of neuroblastoma	Recordati Netherlands B.V.	08 Nov 2012	Qarziba - EU/1/17/1191 - 11 May 2017
EU/3/17/1936	1-[4-bromo-5-[1-ethyl-7-(methylamino)-2-oxo-1,2-dihydro-1,6-naphthyridin-3-yl]-2-fluorophenyl]-3-phenylurea	Treatment of gastrointestinal stromal tumours	Deciphera Pharmaceuticals (Netherlands) B.V.	08 Nov 2017	QINLOCK - EU/1/21/1569 - 24 Nov 2021
EU/3/10/738	Glyceryl tri-(4-phenylbutyrate)	Treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome)	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/737	Glyceryl tri-(4-phenylbutyrate)	Treatment of hyperargininaemia	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/736	Glyceryl tri-(4-phenylbutyrate)	Treatment of argininosuccinic aciduria	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/735	Glyceryl tri-(4-phenylbutyrate)	Treatment of citrullinaemia type 1	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/734	Glyceryl tri-(4-phenylbutyrate)	Treatment of ornithine carbamoyltransferase deficiency	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/733	Glyceryl tri-(4-phenylbutyrate)	Treatment of carbamoyl-phosphate synthase-1 deficiency	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/07/434	Idebenone	Treatment of Leber's hereditary optic neuropathy	Chiesi Farmaceutici S.p.A.	15 Feb 2007	Raxone - EU/1/15/1020 - 10 Sep 2015

EU/3/14/1331	Recombinant fusion protein consisting of a modified form of the extracellular domain of human activin receptor IIB linked to the human IgG1 Fc domain	Treatment of myelodysplastic syndromes	Bristol-Myers Squibb Pharma EEIG	22 Aug 2014	Reblozyl - EU/1/20/1452 - 26 Jun 2020
EU/3/14/1300	Recombinant fusion protein consisting of a modified form of the extracellular domain of human activin receptor IIB linked to the human IgG1 Fc domain	Treatment of beta-thalassaemia intermedia and major	Bristol-Myers Squibb Pharma EEIG	29 Jul 2014	Reblozyl - EU/1/20/1452 - 26 Jun 2020
EU/3/01/077	[gly2]-recombinant human glucagon-like peptide	Treatment of Short Bowel Syndrome	Takeda Pharmaceuticals International AG Ireland Branch	11 Dec 2001	Revestive - EU/1/12/787 - 04 Sep 2012
EU/3/20/2385	Rezafungin acetate	Treatment of invasive candidiasis	Mundipharma GmbH	06 Jan 2021	Rezzayo - EU/1/23/1775 - 22 Dec 2023
EU/3/16/1622	Adeno-associated viral vector serotype 5 containing a B-domain deleted variant of human coagulation factor VIII gene	Treatment of haemophilia A	BioMarin International Limited	21 Mar 2016	Roctavian - EU/1/22/1668 - 25 Aug 2022
EU/3/10/765	Midostaurin	Treatment of mastocytosis	Novartis Europharm Limited	04 Aug 2010	Rydapt - EU/1/17/1218 - 20 Sep 2017
EU/3/04/214	Midostaurin	Treatment of acute myeloid leukaemia	Novartis Europharm Limited	29 Jul 2004	Rydapt - EU/1/17/1218 - 20 Sep 2017
EU/3/20/2272	Rozanolixizumab	Treatment of myasthenia gravis	UCB Pharma	22 Apr 2020	Rystiggo - EU/1/23/1780 - 08 Jan 2024
EU/3/20/2261	Asciminib	Treatment of chronic myeloid leukaemia	Novartis Europharm Limited	24 Mar 2020	Scemblix - EU/1/22/1670 - 26 Aug 2022
EU/3/08/541	[Nle4, D-Phe7]-alpha-melanocyte stimulating hormone	Treatment of erythropoietic protoporphyria	Clinuvel Europe Limited	08 May 2008	SCENESSE - EU/1/14/969 - 29 Dec 2014
EU/3/09/670	pasireotide	Treatment of acromegaly	Recordati Rare Diseases	08 Oct 2009	Signifor - EU/1/12/753 - 21 Nov 2014
EU/3/05/314	(1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethanol	Treatment of tuberculosis	Janssen-Cilag International NV	26 Aug 2005	Sirturo - EU/1/13/901 - 07 Mar 2014

EU/3/18/2037	Omaveloxolone	Treatment of Friedreich's ataxia	Reata Ireland Limited	27 Jun 2018	Skyclarys - EU/1/23/1786 - 12 Feb 2024
EU/3/19/2213	Lonapegsomatropin	Treatment of growth hormone deficiency	Ascendis Pharma Endocrinology Division A/S	17 Oct 2019	Skytrofa - EU/1/21/1607 - 17 Jan 2022
EU/3/18/2068	Somapacitan	Treatment of growth hormone deficiency	Novo Nordisk A/S	24 Aug 2018	Sogroya - EU/1/20/1501 - 06 Apr 2021
EU/3/14/1304	Eculizumab	Treatment of myasthenia gravis	Alexion Europe SAS	29 Jul 2014	Soliris - EU/1/07/393 - 17 Aug 2017
EU/3/13/1185	Eculizumab	Treatment of neuromyelitis optica spectrum disorders	Alexion Europe SAS	05 Aug 2013	Soliris - EU/1/07/393 - 28 Aug 2019
EU/3/15/1450	Gallium (68Ga)-edotreotide	Diagnosis of gastro-entero-pancreatic neuroendocrine tumours	Advanced Accelerator Applications	19 Mar 2015	SomaKit TOC - EU/1/16/1141 - 12 Dec 2016
EU/3/20/2374	Trametinib dimethyl sulfoxide	Treatment of glioma	Novartis Europharm Limited	09 Dec 2020	Spexotras - EU/1/23/1781 - 08 Jan 2024
EU/3/12/976	Antisense oligonucleotide targeted to the SMN2 gene	Treatment of 5q spinal muscular atrophy	Biogen Netherlands B.V.	02 Apr 2012	Spinraza - EU/1/17/1188 - 01 Jun 2017
EU/3/08/594	Recombinant human tissue non-specific alkaline phosphatase - Fc - deca-aspartate fusion protein	Treatment of hypophosphatasia	Alexion Europe SAS	03 Dec 2008	Strensiq - EU/1/15/1015 - 01 Sep 2015
EU/3/05/313	Autologous CD34+ cells transfected with retroviral vector containing adenosine deaminase gene	Treatment of severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency	Fondazione Telethon Ets	26 Aug 2005	Strimvelis - EU/1/16/1097 - 30 May 2016
EU/3/07/508	Chimeric-anti-interleukin-6 monoclonal antibody	Treatment of Castleman's disease	Recordati Netherlands B.V.	30 Nov 2007	Sylvant - EU/1/14/928 - 27 May 2014
EU/3/17/1828	1-(2,2-difluoro-2H-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl}cyclopropane-1-carboxamide and ivacaftor	Treatment of cystic fibrosis	Vertex Pharmaceuticals (Ireland) Limited	27 Feb 2017	Symkevi - EU/1/18/1306 - 06 Nov 2018

EU/3/15/1551	Recombinant human IgG1 kappa light chain monoclonal antibody targeting plasma kallikrein	Treatment of hereditary angioedema	Takeda Pharmaceuticals International AG Ireland Branch	09 Oct 2015	TAKHZYRO - EU/1/18/1340 - 26 Nov 2018
EU/3/21/2486	Talquetamab	Treatment of multiple myeloma	Janssen-Cilag International N.V.	20 Aug 2021	TALVEY - EU/1/23/1748 - 22 Aug 2023
EU/3/14/1373	(2R,3S)-2-(4-cyclopentylaminophenyl)-1-(2-fluoro-6-methylbenzoyl)piperidine-3-carboxylic acid(4-methyl-3-trifluoromethylphenyl)amide	Treatment of granulomatosis with polyangiitis	Vifor Fresenius Medical Care Renal Pharma France	19 Nov 2014	Tavneos - EU/1/21/1605 - 19 Jan 2022
EU/3/14/1372	(2R,3S)-2-(4-cyclopentylaminophenyl)-1-(2-fluoro-6-methylbenzoyl)piperidine-3-carboxylic acid(4-methyl-3-trifluoromethylphenyl)amide	Treatment of microscopic polyangiitis	Vifor Fresenius Medical Care Renal Pharma France	19 Nov 2014	Tavneos - EU/1/21/1605 - 19 Jan 2022
EU/3/20/2344	Autologous peripheral blood T cells CD4 and CD 8 selected and CD 3 and CD 28 activated transduced with retroviral vector expressing anti- CD 19 CD 28/ CD 3-zeta chimeric antigen receptor and cultured	Treatment of acute lymphoblastic leukaemia	Kite Pharma EU B.V.	19 Oct 2020	Tecartus - EU/1/20/1492 - 05 Sep 2022
EU/3/19/2220	Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti CD19 CD28/CD3-zeta chimeric antigen receptor and cultured	Treatment of mantle cell lymphoma	Kite Pharma EU B.V.	13 Nov 2019	Tecartus - EU/1/20/1492 - 15 Dec 2020
EU/3/14/1250	Phosphorothioate oligonucleotide targeted to transthyretin	Treatment of ATTR amyloidosis	Akcea Therapeutics Ireland Limited	26 Mar 2014	Tegsedi - EU/1/18/1296 - 10 Jul 2018
EU/3/22/2581	Epcoritamab	Treatment of diffuse large B-cell lymphoma	AbbVie Deutschland GmbH & Co. KG	24 Feb 2022	Tepkinly - EU/1/23/1759 - 25 Sep 2023
EU/3/18/1994	Ivosidenib	Treatment of biliary tract cancer	Les Laboratoires Servier	21 Mar 2018	Tibsovo - EU/1/23/1728 - 08 May 2023
EU/3/16/1802	Ivosidenib	Treatment of acute myeloid leukaemia	Les Laboratoires Servier	12 Dec 2016	Tibsovo - EU/1/23/1728 - 08 May 2023
EU/3/05/278	(3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid	Treatment of Duchenne muscular dystrophy	PTC Therapeutics International Limited	27 May 2005	Translarna - EU/1/13/902 - 05 Aug 2014

EU/3/04/186	Treosulfan	Conditioning treatment prior to haematopoietic progenitor cell transplantation	medac Gesellschaft für klinische Spezialpräparate mbH	23 Feb 2004	Trecondi - EU/1/18/1351 - 24 Jun 2019
EU/3/13/1103	Treprostinil sodium	Treatment of chronic thromboembolic pulmonary hypertension	SciPharm Sàrl	08 Feb 2013	Trepulmix - EU/1/19/1419 - 07 Apr 2020
EU/3/16/1786	Recombinant adeno-associated viral vector serotype 2 carrying the gene for the human aromatic L-amino acid decarboxylase protein	Treatment of aromatic L-amino acid decarboxylase deficiency	PTC Therapeutics International Limited	18 Nov 2016	Upstaza - EU/1/22/1653 - 22 Jul 2022
EU/3/06/360	Ciclosporin	Treatment of vernal keratoconjunctivitis	Santen Oy	06 Apr 2006	Verkazia - EU/1/17/1219 - 10 Jul 2018
EU/3/09/657	Recombinant human N-acetylgalactosamine-6-sulfatase	Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)	BioMarin International Limited	24 Jul 2009	Vimizim - EU/1/14/914 - 30 Apr 2014
EU/3/02/128	Carboxypeptidase G2	Adjunctive treatment in patients at risk of methotrexate toxicity	Serb	03 Feb 2003	Voraxaze - EU/1/21/1586 - 13 Jan 2022
EU/3/12/1094	Modified recombinant human C-type natriuretic peptide	Treatment of achondroplasia	BioMarin International Limited	24 Jan 2013	Voxzogo - EU/1/21/1577 - 01 Sep 2021
EU/3/18/1992	Efgartigimod alfa	Treatment of myasthenia gravis	Argenx	21 Mar 2018	Vyvgart - EU/1/22/1674 - 11 Aug 2022
EU/3/11/942	Liposomal combination of cytarabine and daunorubicin	Treatment of acute myeloid leukaemia	Jazz Pharmaceuticals Ireland Ltd	11 Jan 2012	Vyxeos liposomal - EU/1/18/1308 - 27 Aug 2018
EU/3/07/459	1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride	Treatment of narcolepsy	Bioprojet Pharma	10 Jul 2007	Wakix - EU/1/15/1068 - 04 Apr 2016
EU/3/14/1249	Phosphorothioate oligonucleotide targeted to apolipoprotein C-III	Treatment of familial chylomicronemia syndrome	Akcea Therapeutics Ireland Limited	19 Feb 2014	Waylivra - EU/1/19/1360 - 08 May 2019
EU/3/01/056	Recombinant human acid sphingomyelinase	Treatment of Niemann-Pick disease	Sanofi B.V.	19 Sep 2001	Xenpozyme - EU/1/22/1659 - 27 Jun 2022

EU/3/09/661	(S)-ethyl 2-amino-3-(4-(2-amino-6-((R)-1-(4-chloro-2-(3-methyl-1H-pyrazol-1-yl)phenyl)-2,2,2-trifluoroethoxy)pyrimidin-4-yl)phenyl)propanoate	Treatment of carcinoid syndrome	Serb	08 Oct 2009	Xermelo - EU/1/17/1224 - 20 Sep 2017
EU/3/17/1961	Gilteritinib	Treatment of acute myeloid leukaemia	Astellas Pharma Europe B.V.	17 Jan 2018	Xospata - EU/1/19/1399 - 28 Oct 2019
EU/3/15/1579	Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor	Treatment of follicular lymphoma	Kite Pharma EU B.V.	11 Nov 2015	Yescarta - EU/1/18/1299 - 23 Jun 2022
EU/3/15/1553	Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor	Treatment of primary mediastinal large B-cell lymphoma	Kite Pharma EU B.V.	09 Oct 2015	Yescarta - EU/1/18/1299 - 27 Aug 2018
EU/3/14/1393	Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3 zeta chimeric antigen receptor	Treatment of diffuse large B cell lymphoma	Kite Pharma EU B.V.	16 Dec 2014	Yescarta - EU/1/18/1299 - 27 Aug 2018
EU/3/20/2350	Poly(oxy-1,2-ethanediyl), alpha-hydro-omega-methoxy, ether with N-[[[2-[[6-[[1-[3-[[3-(2,3-dihydroxypropoxy)propyl]amino]-3-oxopropyl]-2,5-dioxo-3-pyrrolidinyl]thio]hexyl]amino]ethyl]amino]carbonyl]-2-methylalanyl-teriparatide (2:1)	Treatment of hypoparathyroidism	Ascendis Pharma Bone Diseases A/S	19 Oct 2020	Yorvipath - EU/1/23/1766 - 20 Nov 2023
EU/3/10/760	(3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt	Treatment of ovarian cancer	GlaxoSmithKline (Ireland) Limited	04 Aug 2010	Zejula - EU/1/17/1235 - 20 Nov 2017
EU/3/18/2118	Lonafarnib	Treatment of Hutchinson-Gilford progeria syndrome	EigerBio Europe Limited	14 Dec 2018	Zokinvy - EU/1/22/1660 - 19 Jul 2022
EU/3/15/1509	Adeno-associated viral vector serotype 9 containing the human SMN gene	Treatment of spinal muscular atrophy	Novartis Europharm Limited	19 Jun 2015	Zolgensma - EU/1/20/1443 - 18 May 2020
EU/3/19/2224	Ganaxolone	Treatment of CDKL5 deficiency disorder	Marinus Pharmaceuticals Emerald Limited	13 Nov 2019	ZTALMY - EU/1/23/1743 - 28 Jul 2023

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