Lista lekova za retke bolesti-18.11.2026

EU broj	Proizvod / aktivna supstanca	Indikacija	Sponzor	Datum određivanja	Trgovački naziv – EU broj proizvoda – datum
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	Lečenje multiplog mijeloma	Bristol-Myers Squibb Pharma EEIG	20 Apr 2017	Abecma - EU/1/21/1539 - 19 Aug 2021
EU/3/08/588	Recombinant human ADAMTS-13	Lečenje trombotičke trombocitopenijske purpure	Takeda Manufacturing Austria AG	03 Dec 2008	ADZYNMA - EU/1/24/1837 - 02 Aug 2024
EU/3/14/1309	17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione	Lečenje Dišenove mišićne distrofije	Santhera Pharmaceuticals (Deutschland) GmbH	22 Aug 2014	AGAMREE - EU/1/23/1776 - 15 Dec 2023
EU/3/07/498	Polihexanide	Lečenje akantamebnog keratitisa	S.I.F.I. Società Industria Farmaceutica Italiana S.p.A.	14 Nov 2007	AKANTIOR - EU/1/24/1840 - 23 Aug 2024
EU/3/07/453	Recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1	Lečenje hemofilije B (urođeni nedostatak faktora IX)	Swedish Orphan Biovitrum AB (publ)	08 Jun 2007	ALPROLIX - EU/1/16/1098 - 13 May 2016
EU/3/19/2176	Recombinant human coagulation factor VIII Fc - von Willebrand factor - XTEN fusion protein	Lečenje hemofilije A	Swedish Orphan Biovitrum AB (publ)	28 Jun 2019	ALTUVOCT - EU/1/24/1824 - 18 Jun 2024
EU/3/21/2527	(14S)-8-[3-(2-{dispiro[2.0.24.13]heptan-7-yl}ethoxy)-1H-pyrazol-1-yl]-12,12-dimethyl-2lamba6-thia-3,9,11,18,23-penta-azatetracyclo[17.3.1.111,14.05,10]tetracosa-1(22),5,7,9,19(23),20-hexaene-2,2,4-trione calcium salt hydrate, deutivacaftor, tezacaftor	Lečenje cistične fibroze	Vertex Pharmaceuticals (Ireland) Limited	12 Nov 2021	Alyftrek - EU/1/25/1943 - 01 Jul 2025
EU/3/15/1589	Glibenclamide	Lečenje neonatalnog dijabetesa	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	Lečenje amiloidoze posredovane transtiretinom	Alnylam Netherlands B.V.	25 May 2018	Amvuttra - EU/1/22/1681 - 16 Sep 2022
EU/3/17/1848	Acetylleucine	Lečenje Nimann-Pikove bolesti	IntraBio Ireland Ltd	20 Mar 2017	Aqneursa - EU/1/25/1928 - 20 Jan 2026
EU/3/14/1259	Amikacin sulfate	Lečenje plućne bolesti izazvane netuberkuloznim mikobakterijama	Insmed Netherlands B.V.	08 Apr 2014	ARIKAYCE liposomal - EU/1/20/1469 - 28 Oct 2020
EU/3/20/2251	Artesunate	Lečenje malarije	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-glutaminyl-L-.alpha.-aspartyl-L-tryptophylglycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-L-threonyl-2-[2-(2-aminoethoxy)ethoxy]acetyl-N6-carboxy-L-lysinamide cyclic (2.fwdarw.12)-(disulfide); where two identical synthetic peptide domains are covalently linked at the ends of the polyethylene glycol chain	Lečenje paroksizmalne noćne hemoglobinurije	Swedish Orphan Biovitrum AB (publ)	22 May 2017	ASPAVELI - EU/1/21/1595 - 14 Dec 2021
EU/3/22/2716	Pegcetacoplan	Lečenje C3 glomerulopatije sa imunskim kompleksima ili bez njih	Swedish Orphan Biovitrum AB (publ)	10 Nov 2022	ASPAVELI - EU/1/21/1595 - 16 Jan 2026
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	Lečenje gastrointestinalnih stromalnih tumora	Sanofi-Aventis Ireland Limited	17 Jul 2017	AYVAKYT - EU/1/20/1473 - 25 Sep 2020

EU/3/18/2074	Avapritinib	Lečenje mastocitoze	Sanofi-Aventis Ireland Limited	26 Oct 2018	AYVAKYT - EU/1/20/1473 - 25 Mar 2022
EU/3/13/1127	Inotuzumab ozogamicin	Lečenje akutne limfoblastne leukemije B-ćelija	Pfizer Europe MA EEIG	07 Jun 2013	Besponsa - EU/1/17/1200 - 03 Jul 2017
EU/3/09/650	Blinatumomab	Lečenje akutne limfoblastne leukemije	Amgen Europe B.V.	24 Jul 2009	BLINCYTO - EU/1/15/1047 - 25 Nov 2015
EU/3/13/1118	Recombinant human tripeptidyl-peptidase 1	Lečenje neuronalne ceroidne lipofuscinoze tipa 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	Lečenje progresivne porodične intrahepatične holestaze	Ipsen Pharma	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	Lečenje trombotičke trombocitopenijske purpure	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	Lečenje multiplog mijeloma	Janssen-Cilag International NV	28 Feb 2020	CARVYKTI - EU/1/22/1648 - 30 May 2022
EU/3/19/2210	Autologous CD34+ haematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene	Lečenje beta-talasemije intermedije i major oblika	Vertex Pharmaceuticals (Ireland) Limited	17 Oct 2019	Casgevy - EU/1/23/1787 - 12 Feb 2024
EU/3/19/2242	Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene	Lečenje bolesti srpastih ćelija	Vertex Pharmaceuticals (Ireland) Limited	09 Jan 2020	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	Lečenje Gošeove bolesti	Sanofi B.V.	04 Dec 2007	Cerdelga - EU/1/14/974 - 21 Jan 2015
EU/3/14/1406	Chenodeoxycholic acid	Lečenje urođenih poremećaja sinteze primarnih žučnih kiselina	Leadiant Biosciences Ireland Limited	16 Dec 2014	Chenodeoxycholic acid Leadiant - EU/1/16/1110 - 12 Apr 2017

EU/3/07/471	Human coagulation factor X	Lečenje naslednog nedostatka faktora X	Kedrion S.p.A.	17 Sep 2007	Coagadex - EU/1/16/1087 - 18 Mar 2016
EU/3/14/1276	Isavuconazonium sulfate	Lečenje mukormikoze	Basilea Pharmaceutica Deutschland GmbH	04 Jun 2014	Cresemba - EU/1/15/1036 - 19 Oct 2015
EU/3/14/1284	Isavuconazonium sulfate	Lečenje invazivne aspergiloze	Basilea Pharmaceutica Deutschland GmbH	04 Jul 2014	Cresemba - EU/1/15/1036 - 19 Oct 2015
EU/3/14/1351	Recombinant human monoclonal IgG1 antibody for fibroblast growth factor 23	Lečenje X-vezane hipofosfatemije	Kyowa Kirin Holdings B.V.	15 Oct 2014	CRYSVITA - EU/1/17/1262 - 21 Feb 2018
EU/3/18/2011	Burosumab	Lečenje fosfaturičnog mezenhimalnog tumora	Kyowa Kirin Holdings B.V.	16 Apr 2018	CRYSVITA - EU/1/17/1262 - 28 Jul 2022
EU/3/08/578	Cysteamine hydrochloride	Lečenje cistinoze	Recordati Rare Diseases	07 Nov 2008	Cystadrops - EU/1/15/1049 - 23 Jan 2017
EU/3/17/1923	Glasdegib maleate	Lečenje akutne mijeloidne leukemije	Pfizer Europe MA EEIG	16 Oct 2017	Daurismo - EU/1/20/1451 - 29 Jun 2020
EU/3/15/1534	Glycyl-L-2-methylprolyl-L-glutamic acid	Lečenje Retovog sindroma	Acadia Pharmaceuticals (Netherlands) B.V.	10 Aug 2015	Daybu - EU/1/26/2045 - 21 Aug 2026
EU/3/07/513	(S)-2-nitro-6-(4-(trifluoromethoxy)benzyloxy)-6,7-dihydro-5H-imidazo[2,1-b][1,3]oxazine	Lečenje tuberkuloze	Viatris Healthcare Limited	29 Nov 2007	Dovprela - EU/1/20/1437 - 04 Aug 2020
EU/3/12/1009	Givinostat	Lečenje Dišenove mišićne distrofije	Italfarmaco S.p.A.	04 Jul 2012	DUVYZAT - EU/1/25/1930 - 06 Jun 2025
EU/3/16/1627	Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes	Lečenje posttransplantacionog limfoproliferativnog poremećaja	Pierre Fabre Medicament	21 Mar 2016	Ebvallo - EU/1/22/1700 - 16 Dec 2022
EU/3/22/2625	Sebetralstat	Lečenje naslednog angioedema	Kalvista Pharmaceuticals (Ireland) Limited	21 Jun 2022	Ekterly - EU/1/25/1975 - 18 Sep 2025
EU/3/15/1458	Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4	Lečenje karcinoma jajnika	AbbVie Deutschland GmbH & Co. KG	19 Mar 2015	ELAHERE - EU/1/24/1866 - 15 Nov 2024

EU/3/15/1567	Recombinant human interleukin-3 truncated diphtheria toxin fusion protein	Lečenje blastične neoplazme plazmacitoidnih dendritskih ćelija	Stemline Therapeutics B.V.	11 Nov 2015	ELZONRIS - EU/1/20/1504 - 13 Jan 2021
EU/3/17/1945	Tiratricol	Lečenje Alan-Herndon-Dadlijevog sindroma	Rare Thyroid Therapeutics International AB	08 Nov 2017	Emcitate - EU/1/24/1897 - 17 Feb 2025
EU/3/16/1609	Humanised IgG4 monoclonal antibody against total complement component 1, subcomponent s	Lečenje autoimune hemolitičke anemije	Recordati Rare Diseases	17 Feb 2016	Enjaymo - EU/1/22/1687 - 23 Nov 2022
EU/3/16/1680	Humanised anti-IL-6 receptor monoclonal antibody	Lečenje poremećaja iz spektra optičkog neuromijelitisa	Roche Registration GmbH	27 Jun 2016	Enspryng - EU/1/21/1559 - 29 Jun 2021
EU/3/14/1339	Cannabidiol	Lečenje Draveovog sindroma	Jazz Pharmaceuticals Ireland Limited	15 Oct 2014	Epidyolex - EU/1/19/1389 - 23 Sep 2019
EU/3/17/1855	Cannabidiol	Lečenje Lenoks-Gastoovog sindroma	Jazz Pharmaceuticals Ireland Limited	20 Mar 2017	Epidyolex - EU/1/19/1389 - 23 Sep 2019
EU/3/17/1959	Cannabidiol	Lečenje tuberozne skleroze	Jazz Pharmaceuticals Ireland Limited	17 Jan 2018	Epidyolex - EU/1/19/1389 - 20 Apr 2021
EU/3/19/2184	N-((R)-2,3-dihydroxypropoxyl)-3,4-difluro-2-(2-fluoro-4-iodo-phenylamino)-benzamide	Lečenje neurofibromatoze tipa 1	Merck Europe B.V.	25 Jul 2019	Ezmekly - EU/1/25/1950 - 18 Jul 2025
EU/3/18/2104	(4-{(2S,4S)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl}benzoic acid-hydrogen chloride(1/1))	Lečenje C3 glomerulopatije	Novartis Europharm Limited	14 Dec 2018	FABHALTA - EU/1/24/1802 - 01 Apr 2025
EU/3/20/2281	(4-{(2S,4S)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl}benzoic acid-hydrogen chloride(1/1))	Lečenje paroksizmalne noćne hemoglobinurije	Novartis Europharm Limited	04 Jun 2020	FABHALTA - EU/1/24/1802 - 21 May 2024
EU/3/20/2345	Sparsentan	Lečenje primarne IgA nefropatije	Vifor France S.A.	19 Oct 2020	Filspari - EU/1/23/1788 - 23 Apr 2024
EU/3/10/845	Dry extract from birch bark (DER 5-10 : 1), extraction solvent n-heptane 95% (w/w)	Lečenje bulozne epidermolize	Chiesi Farmaceutici S.p.A.	23 Feb 2011	Filsuvez - EU/1/22/1652 - 23 Jun 2022
EU/3/20/2372	Dabrafenib mesylate	Lečenje glioma	Novartis Europharm Limited	09 Dec 2020	Finlee - EU/1/23/1767 - 16 Nov 2023

EU/3/13/1219	Fenfluramine hydrochloride	Lečenje Draveovog sindroma	UCB Pharma	18 Dec 2013	Fintepla - EU/1/20/1491 - 21 Dec 2020
EU/3/17/1836	Fenfluramine hydrochloride	Lečenje Lenoks-Gastoovog sindroma	UCB Pharma	27 Feb 2017	Fintepla - EU/1/20/1491 - 31 Jan 2023
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	Lečenje akutne hepatične porfirije	Alnylam Netherlands B.V.	29 Aug 2016	Givlaari - EU/1/20/1428 - 04 Mar 2020
EU/3/18/1999	Recombinant adeno-associated viral vector containing a codon-optimized Padua derivative of human coagulation factor IX cDNA	Lečenje hemofilije 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	Lečenje infekcije virusom hepatitisa delta	Gilead Sciences Ireland Unlimited Company	19 Jun 2015	Hepcludex - EU/1/20/1446 - 05 Aug 2020
EU/3/08/579	Ex-vivo expanded autologous human corneal epithelium containing stem cells	Lečenje lezija rožnjače povezanih sa nedostatkom matičnih ćelija rožnjače (limbusa), usled opekotina oka	Holostem S.r.l.	07 Nov 2008	Holoclar - EU/1/14/987 - 19 Feb 2015
EU/3/17/1910	Sirolimus	Lečenje tuberozne skleroze	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	Prevencija odbacivanja grafta nakon transplantacije solidnih organa	Serb	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	Lečenje hemofilije B	CSL Behring GmbH	04 Feb 2010	IDELVION - EU/1/16/1095 - 13 May 2016
EU/3/16/1703	Setmelanotide	Lečenje nedostatka proopiomelanokortina	Rhythm Pharmaceuticals Netherlands B.V.	14 Jul 2016	Imcivree - EU/1/21/1564 - 19 Jul 2021
EU/3/18/2101	Setmelanotide	Lečenje nedostatka receptora za leptin	Rhythm Pharmaceuticals Netherlands B.V.	19 Nov 2018	Imcivree - EU/1/21/1564 - 19 Jul 2021
EU/3/19/2192	Setmelanotide	Lečenje Barde-Bidl sindroma	Rhythm Pharmaceuticals Netherlands B.V.	21 Aug 2019	Imcivree - EU/1/21/1564 - 05 Sep 2022

EU/3/23/2833	Setmelanotide	Lečenje stečene hipotalamičke gojaznosti	Rhythm Pharmaceuticals Netherlands B.V.	13 Oct 2023	Imcivree - EU/1/21/1564 - 05 May 2026
EU/3/23/2876	Tarlatamab	Lečenje neuroendokrinog karcinoma pluća (prvobitno nazvano lečenje mikrocelularnog karcinoma pluća)	Amgen Europe B.V.	12 Jan 2024	IMDYLLTRA - EU/1/26/2033 - 01 Jun 2026
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	Lečenje primarne mijelofibroze	Bristol-Myers Squibb Pharma EEIG	01 Oct 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	Lečenje mijelofibroze nakon esencijalne trombocitemije	Bristol-Myers Squibb Pharma EEIG	26 Nov 2010	Inrebic - EU/1/20/1514 - 09 Feb 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	Lečenje mijelofibroze nakon policitemije vere	Bristol-Myers Squibb Pharma EEIG	26 Nov 2010	Inrebic - EU/1/20/1514 - 09 Feb 2021
EU/3/19/2182	Elafibranor	Lečenje primarnog bilijarnog holangitisa	Ipsen Pharma	25 Jul 2019	Iqirvo - EU/1/24/1855 - 23 Sep 2024
EU/3/14/1345	Osilodrostat	Lečenje Kušingovog sindroma	Recordati Rare Diseases	15 Oct 2014	Isturisa - EU/1/19/1407 - 13 Jan 2020
EU/3/20/2339	Leniolisib	Lečenje sindroma aktivirane fosfoinozitid 3-kinaze delta	Pharming Technologies B.V.	19 Oct 2020	Joenja - EU/1/26/2034 - 22 May 2026
EU/3/13/1181	Budesonide	Lečenje eozinofilnog ezofagitisa	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	Lečenje cistične fibroze	Vertex Pharmaceuticals (Ireland) Limited	14 Dec 2018	Kaftrio - EU/1/20/1468 - 21 Aug 2020

EU/3/10/827	Recombinant human lysosomal acid lipase	Lečenje nedostatka lizozomalne kisele lipaze	Alexion Europe SAS	17 Dec 2010	Kanuma - EU/1/15/1033 - 01 Sep 2015
EU/3/21/2397	Tebentafusp	Lečenje uvealnog melanoma	Immunocore Ireland Limited	19 Feb 2021	KIMMTRAK - EU/1/22/1630 - 06 Apr 2022
EU/3/16/1778	Budesonide	Lečenje primarne IgA nefropatije	STADA Arzneimittel AG	18 Nov 2016	Kinpeygo - EU/1/22/1657 - 18 Jul 2022
EU/3/18/2050	Selumetinib	Lečenje neurofibromatoze tipa 1	AstraZeneca AB	31 Jul 2018	Koselugo - EU/1/21/1552 - 19 Jun 2021
EU/3/17/1870	Thymidine and deoxycytidine	Lečenje sindroma deplecije mitohondrijalne DNK, miopatski oblik	UCB Pharma	20 Apr 2017	KYGEVVI - EU/1/25/2013 - 27 Mar 2026
EU/3/14/1266	Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19	Lečenje B-limfoblastne leukemije/limfoma	Novartis Europharm Limited	29 Apr 2014	Kymriah - EU/1/18/1297 - 27 Aug 2018
EU/3/16/1745	Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19	Lečenje difuznog B-krupnoćelijskog limfoma	Novartis Europharm Limited	14 Oct 2016	Kymriah - EU/1/18/1297 - 27 Aug 2018
EU/3/21/2464	Tisagenlecleucel	Lečenje folikularnog limfoma	Novartis Europharm Limited	19 Jul 2021	Kymriah - EU/1/18/1297 - 03 May 2022
EU/3/04/260	Recombinant human α-Mannosidase	Lečenje alfa-manozidoze	Chiesi Farmaceutici S.p.A.	26 Jan 2005	Lamzede - EU/1/17/1258 - 27 Mar 2018
EU/3/12/963	Chlormethine	Lečenje kožnog T-ćelijskog limfoma	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	Lečenje metahromatske leukodistrofije	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	Lečenje Alažilovog sindroma	Mirum Pharmaceuticals International B.V.	18 Dec 2013	Livmarli - EU/1/22/1704 - 12 Dec 2022

EU/3/13/1216	(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	Lečenje progresivne porodične intrahepatične holestaze	Mirum Pharmaceuticals International B.V.	18 Dec 2013	Livmarli - EU/1/22/1704 - 01 Jul 2024
EU/3/07/519	Maribavir	Prevencija citomegalovirusne (CMV) bolesti kod pacijenata sa oslabljenim ćelijski posredovanim imunitetom za koje se smatra da su pod rizikom	Takeda Pharmaceuticals International AG Ireland Branch	18 Dec 2007	LIVTENCITY - EU/1/22/1672 - 10 Nov 2022
EU/3/13/1133	Maribavir	Lečenje citomegalovirusne bolesti kod pacijenata sa oslabljenim ćelijski posredovanim imunitetom	Takeda Pharmaceuticals International AG Ireland Branch	07 Jun 2013	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	Lečenje hiperargininemije	Immedica Pharma AB	14 Jul 2016	Loargys - EU/1/23/1774 - 18 Dec 2023
EU/3/21/2517	Mosunetuzumab	Lečenje folikularnog limfoma	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	Lečenje gastroenteropankreatičnih neuroendokrinih tumora	Novartis Europharm Limited	31 Jan 2008	Lutathera - EU/1/17/1226 - 28 Sep 2017
EU/3/12/981	Adenovirus associated viral vector serotype 2 containing the human RPE65 gene	Lečenje naslednih distrofija mrežnjače (prvobitno nazvano lečenje Leberove kongenitalne amauroze)	Novartis Europharm Limited	02 Apr 2012	Luxturna - EU/1/18/1331 - 05 Dec 2018
EU/3/15/1518	Adenovirus-associated viral vector serotype 2 containing the human RPE65 gene	Lečenje naslednih distrofija mrežnjače (prvobitno nazvano lečenje retinitis pigmentoze)	Novartis Europharm Limited	28 Jul 2015	Luxturna - EU/1/18/1331 - 05 Dec 2018
EU/3/21/2515	Linerixibat	Lečenje primarnog bilijarnog holangitisa	GlaxoSmithKline Trading Services Limited	12 Nov 2021	Lynavoy - EU/1/26/2053 - 30 Oct 2026
EU/3/17/1930	Seladelpar	Lečenje primarnog bilijarnog holangitisa	Gilead Sciences Ireland UC	16 Oct 2017	Lyvdelzi - EU/1/24/1898 - 21 Feb 2025

EU/3/18/2076	Glycine, L-alanine, L-arginine, L-aspartic acid, L-cysteine, L-glutamic acid, L-histidine, L-lysine monohydrate, L-methionine, L-phenylalanine, L-proline, L-serine, L-threonine, L-tryptophan, L-tyrosine, taurine	Lečenje bolesti urina sa mirisom javorovog sirupa	Recordati Rare Diseases	26 Oct 2018	Maapliv - EU/1/25/1955 - 29 Jul 2025
EU/3/12/973	Recombinant human beta-glucuronidase	Lečenje mukopolisaharidoze tipa VII (Slajev sindrom)	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	Lečenje difuznog B-krupnoćelijskog limfoma	Incyte Biosciences Distribution B.V.	15 Jan 2015	Minjuvi - EU/1/21/1570 - 01 Sep 2021
EU/3/25/3027	Tafasitamab	Lečenje folikularnog limfoma	Incyte Biosciences Distribution B.V.	26 Feb 2025	Minjuvi - EU/1/21/1570 - 16 Dec 2025
EU/3/12/1022	Metreleptin	Lečenje porodične parcijalne lipodistrofije	Chiesi Farmaceutici S.p.A.	17 Jul 2012	Myalepta - EU/1/18/1276 - 01 Aug 2018
EU/3/12/1023	Metreleptin	Lečenje Baraker-Simonsovog sindroma	Chiesi Farmaceutici S.p.A.	17 Jul 2012	Myalepta - EU/1/18/1276 - 01 Aug 2018
EU/3/12/1024	Metreleptin	Lečenje Lorensovog sindroma	Chiesi Farmaceutici S.p.A.	17 Jul 2012	Myalepta - EU/1/18/1276 - 01 Aug 2018
EU/3/12/1025	Metreleptin	Lečenje Berardineli-Sajpovog sindroma	Chiesi Farmaceutici S.p.A.	17 Jul 2012	Myalepta - EU/1/18/1276 - 01 Aug 2018
EU/3/00/005	Gemtuzumab Ozogamicin	Lečenje akutne mijeloidne leukemije	Pfizer Europe MA EEIG	18 Oct 2000	MYLOTARG - EU/1/18/1277 - 23 Apr 2018
EU/3/14/1353	Mexiletine hydrochloride	Lečenje miotoničnih poremećaja	Lupin Europe GmbH	19 Nov 2014	Namuscla - EU/1/18/1325 - 20 Dec 2018
EU/3/13/1210	Recombinant human parathyroid hormone	Lečenje hipoparatireoidizma	Takeda Pharmaceuticals International AG Ireland Branch	18 Dec 2013	Natpar - EU/1/15/1078 - 26 Apr 2017
EU/3/16/1770	5-[4-[2-(5-(1-hydroxyethyl)-2-pyridinyl)ethoxy]benzyl]-2,4-thiazolidinedione hydrochloride	Lečenje adrenoleukodistrofije	Minoryx Therapeutics S.L.	18 Nov 2016	Nezglyal - EU/1/26/2060 - 22 Sep 2026

EU/3/12/1087	Recombinant modified human growth hormone	Lečenje nedostatka hormona rasta	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	Lečenje multiplog mijeloma	Takeda Pharma A/S	27 Sep 2011	Ninlaro - EU/1/16/1094 - 23 Nov 2016
EU/3/10/777	Cyclic pyranopterin monophosphate	Lečenje nedostatka molibdenskog kofaktora tipa A	Integral Pharma Solutions EU Limited	20 Sep 2010	NULIBRY - EU/1/22/1684 - 16 Sep 2022
EU/3/19/2214	Nirogacestat	Lečenje sarkoma mekih tkiva	Merck Europe B.V.	17 Oct 2019	Ogsiveo - EU/1/25/1932 - 18 Aug 2025
EU/3/21/2434	6-Amino-5-chloro-N-((1R)-1-(5-(((5-chloro-4-(trifluoromethyl)-2-pyridinyl)amino)carbonyl)-2-thiazolyl)ethyl)-4-pyrimidinecarboxamide	Lečenje glioma	Ipsen Pharma	20 May 2021	Ojemda - EU/1/26/2025 - 21 Apr 2026
EU/3/11/886	N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide, dihydrochloride salt	Lečenje mijelofibroze nakon policitemije vere	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	Lečenje mijelofibroze nakon esencijalne trombocitemije	GlaxoSmithKline Trading Services Limited	05 Aug 2011	Omjjara - EU/1/23/1782 - 26 Jan 2024
EU/3/11/888	N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide, dihydrochloride salt	Lečenje primarne mijelofibroze	GlaxoSmithKline Trading Services Limited	05 Aug 2011	Omjjara - EU/1/23/1782 - 26 Jan 2024
EU/3/11/933	Nanoliposomal irinotecan	Lečenje karcinoma pankreasa	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	Lečenje amiloidoze posredovane transtiretinom	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	Lečenje bolesti srpastih ćelija	Pfizer Europe MA EEIG	18 Nov 2016	Oxbryta - EU/1/21/1622 - 15 Feb 2022
EU/3/15/1586	Recombinant human nerve growth factor	Lečenje neurotrofičnog keratitisa	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	Lečenje primarne hiperoksalurije	Alnylam Netherlands B.V.	21 Mar 2016	Oxlumo - EU/1/20/1496 - 23 Nov 2020
EU/3/25/3035	Paltusotine	Lečenje akromegalije	Crinetics Pharmaceuticals Europe GmbH	26 Feb 2025	Palsonify - EU/1/26/2021 - 27 Apr 2026
EU/3/09/708	Pegylated recombinant phenylalanine ammonia lyase	Lečenje hiperfenilalaninemije	BioMarin International Limited	28 Jan 2010	Palynziq - EU/1/19/1362 - 08 May 2019
EU/3/18/2066	Pemigatinib	Lečenje karcinoma bilijarnog trakta	Incyte Biosciences Distribution B.V.	24 Aug 2018	Pemazyre - EU/1/21/1535 - 29 Mar 2021
EU/3/18/2013	Polatuzumab vedotin	Lečenje difuznog B-krupnoćelijskog limfoma	Roche Registration GmbH	16 Apr 2018	Polivy - EU/1/19/1388 - 20 Jan 2020
EU/3/16/1756	Mogamulizumab	Lečenje kožnog T-ćelijskog limfoma	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	Prevencija citomegalovirusne bolesti kod pacijenata sa oslabljenim ćelijski posredovanim imunitetom za koje se smatra da su pod rizikom	Merck Sharp & Dohme B.V.	15 Apr 2011	PREVYMIS - EU/1/17/1245 - 10 Jan 2018
EU/3/20/2270	Mitapivat sulfate	Lečenje nedostatka piruvat-kinaze	Agios Netherlands B.V.	22 Apr 2020	Pyrukynd - EU/1/22/1662 - 10 Nov 2022
EU/3/23/2827	Mitapivat sulfat	Lečenje talasemije intermedije i major oblika (prvobitno nazvano lečenje beta-talasemije intermedije i major oblika)	Agios Netherlands B.V.	13 Oct 2023	Pyrukynd - EU/1/22/1662 - 22 May 2026
EU/3/23/2889	Mitapivat sulfate	Lečenje talasemije intermedije i major oblika (prvobitno nazvano lečenje beta-talasemije intermedije i major oblika)	Agios Netherlands B.V.	12 Jan 2024	Pyrukynd - EU/1/22/1662 - 22 May 2026

EU/3/16/1732	Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid	Lečenje amiotrofične lateralne skleroze	Biogen Netherlands B.V.	29 Aug 2016	Qalsody - EU/1/23/1783 - 03 Jun 2024
EU/3/12/1062	Chimeric monoclonal antibody against GD2	Lečenje 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	Lečenje gastrointestinalnih stromalnih tumora	Deciphera Pharmaceuticals (Netherlands) B.V.	08 Nov 2017	QINLOCK - EU/1/21/1569 - 24 Nov 2021
EU/3/26/3227	daraxonrasib	Lečenje karcinoma pankreasa	Revolution Medicines Netherlands B.V.	20 Apr 2026	Rasonque - EU/1/26/2079 - 16 Nov 2026
EU/3/10/733	Glyceryl tri-(4-phenylbutyrate)	Lečenje nedostatka karbamoil-fosfat sintetaze 1	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/734	Glyceryl tri-(4-phenylbutyrate)	Lečenje nedostatka ornitin-karbamoiltransferaze	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/735	Glyceryl tri-(4-phenylbutyrate)	Lečenje citrulinemije tipa 1	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/736	Glyceryl tri-(4-phenylbutyrate)	Lečenje argininosukcinične acidurije	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/737	Glyceryl tri-(4-phenylbutyrate)	Lečenje hiperargininemije	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
EU/3/10/738	Glyceryl tri-(4-phenylbutyrate)	Lečenje nedostatka ornitin-translokaze (sindrom hiperornitinemije–hiperamonemije–homocitrulinurije, HHH)	Immedica Pharma AB	10 Jun 2010	Ravicti - EU/1/15/1062 - 01 Dec 2015
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	Lečenje beta-talasemije intermedije i major oblika	Bristol-Myers Squibb Pharma EEIG	29 Jul 2014	Reblozyl - EU/1/20/1452 - 26 Jun 2020
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	Lečenje mijelodisplastičnih sindroma	Bristol-Myers Squibb Pharma EEIG	22 Aug 2014	Reblozyl - EU/1/20/1452 - 26 Jun 2020

EU/3/21/2459	Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues	Lečenje sindroma porodične hilomikronemije	Arrowhead Pharmaceuticals Ireland Limited	19 Jul 2021	Redemplo - EU/1/26/2041 - 22 Jun 2026
EU/3/19/2205	2-(3-(4-(1H-indazol-5-ylamino)quinazolin-2-yl)phenoxy)-N-isopropylacetamide-methane sulfonic acid salt	Lečenje bolesti kalema protiv domaćina	Sanofi Winthrop Industrie	17 Oct 2019	REZUROCK - EU/1/26/2015 - 25 Mar 2026
EU/3/20/2385	Rezafungin acetate	Lečenje invazivne kandidijaze	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	Lečenje hemofilije A	BioMarin International Limited	21 Mar 2016	Roctavian - EU/1/22/1668 - 25 Aug 2022
EU/3/19/2227	2-(isopropylamino)-3-methyl-5-(6-methyl-5-((2-(1-methyl-1H-pyrazol-4-yl)pyridin-4-yl)oxy)pyridin-2-yl)pyrimidin-4(3H)-one	Lečenje tenosinovijalnog tumora džinovskih ćelija, lokalizovanog i difuznog tipa	Deciphera Pharmaceuticals (Netherlands) B.V.	16 Dec 2019	ROMVIMZA - EU/1/25/1968 - 24 Sep 2025
EU/3/04/214	Midostaurin	Lečenje akutne mijeloidne leukemije	Novartis Europharm Limited	29 Jul 2004	Rydapt - EU/1/17/1218 - 20 Sep 2017
EU/3/10/765	Midostaurin	Lečenje mastocitoze	Novartis Europharm Limited	04 Aug 2010	Rydapt - EU/1/17/1218 - 20 Sep 2017
EU/3/20/2272	Rozanolixizumab	Lečenje mijastenije gravis	UCB Pharma	22 Apr 2020	Rystiggo - EU/1/23/1780 - 08 Jan 2024
EU/3/20/2305	Imetelstat sodium	Lečenje mijelodisplastičnih sindroma	Geron Netherlands B.V.	27 Jul 2020	Rytelo - EU/1/24/1894 - 11 Mar 2025
EU/3/20/2261	Asciminib	Lečenje hronične mijeloidne leukemije	Novartis Europharm Limited	24 Mar 2020	Scemblix - EU/1/22/1670 - 26 Aug 2022
EU/3/21/2435	(S)-2-amino-6-(2-hydroxypropanoyl)-7,8-dihydropteridin-4(3H)-one	Lečenje hiperfenilalaninemije	PTC Therapeutics International Limited	20 May 2021	Sephience - EU/1/25/1939 - 25 Jun 2025
EU/3/18/2037	Omaveloxolone	Lečenje Fridrajhove ataksije	Biogen Netherlands B.V.	27 Jun 2018	Skyclarys - EU/1/23/1786 - 12 Feb 2024
EU/3/19/2213	Lonapegsomatropin	Lečenje nedostatka hormona rasta	Ascendis Pharma Endocrinology Division A/S	17 Oct 2019	Skytrofa - EU/1/21/1607 - 17 Jan 2022

EU/3/13/1185	Eculizumab	Lečenje poremećaja iz spektra optičkog neuromijelitisa	Alexion Europe SAS	05 Aug 2013	Soliris - EU/1/07/393 - 28 Aug 2019
EU/3/14/1304	Eculizumab	Lečenje mijastenije gravis	Alexion Europe SAS	29 Jul 2014	Soliris - EU/1/07/393 - 17 Aug 2017
EU/3/15/1450	Gallium (68Ga)-edotreotide	Dijagnostika gastroenteropankreatičnih neuroendokrinih tumora	Novartis Europharm Limited	19 Mar 2015	SomaKit TOC - EU/1/16/1141 - 12 Dec 2016
EU/3/20/2374	Trametinib dimethyl sulfoxide	Lečenje 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	Lečenje 5q spinalne mišićne atrofije	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	Lečenje hipofosfatazije	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	Lečenje teške kombinovane imunodeficijencije (SCID) usled nedostatka adenozin-deaminaze (ADA)	Fondazione Telethon Ets	26 Aug 2005	Strimvelis - EU/1/16/1097 - 30 May 2016
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	Lečenje cistične fibroze	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	Lečenje naslednog angioedema	Takeda Pharmaceuticals International AG Ireland Branch	09 Oct 2015	TAKHZYRO - EU/1/18/1340 - 26 Nov 2018
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	Lečenje limfoma ćelija plašta	Kite Pharma EU B.V.	13 Nov 2019	Tecartus - EU/1/20/1492 - 15 Dec 2020
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	Lečenje akutne limfoblastne leukemije	Kite Pharma EU B.V.	19 Oct 2020	Tecartus - EU/1/20/1492 - 05 Sep 2022

EU/3/14/1250	Phosphorothioate oligonucleotide targeted to transthyretin	Lečenje ATTR amiloidoze	Akcea Therapeutics Ireland Limited	26 Mar 2014	Tegsedi - EU/1/18/1296 - 10 Jul 2018
EU/3/16/1802	Ivosidenib	Lečenje akutne mijeloidne leukemije	Les Laboratoires Servier	12 Dec 2016	Tibsovo - EU/1/23/1728 - 08 May 2023
EU/3/18/1994	Ivosidenib	Lečenje karcinoma bilijarnog trakta	Les Laboratoires Servier	21 Mar 2018	Tibsovo - EU/1/23/1728 - 08 May 2023
EU/3/04/186	Treosulfan	Kondiciona terapija pre transplantacije hematopoetskih progenitorskih ćelija	medac Gesellschaft für klinische Spezialpräparate mbH	23 Feb 2004	Trecondi - EU/1/18/1351 - 24 Jun 2019
EU/3/13/1103	Treprostinil sodium	Lečenje hronične tromboembolijske plućne hipertenzije	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	Lečenje nedostatka dekarboksilaze aromatičnih L-aminokiselina	PTC Therapeutics International Limited	18 Nov 2016	Upstaza - EU/1/22/1653 - 22 Jul 2022
EU/3/06/360	Ciclosporin	Lečenje vernalnog keratokonjunktivitisa	Santen Oy	06 Apr 2006	Verkazia - EU/1/17/1219 - 10 Jul 2018
EU/3/21/2420	Alpelisib	Lečenje spektra sindroma prekomernog rasta povezanih sa PIK3CA	Novartis Europharm Limited	26 Mar 2021	Vijoice - EU/1/26/2042 - 16 Jul 2026
EU/3/22/2737	Vorasidenib hemicitrate hemihydrate	Lečenje glioma	Les Laboratoires Servier	13 Jan 2023	Voranigo - EU/1/25/1912 - 19 Sep 2025
EU/3/02/128	Carboxypeptidase G2	Pomoćna terapija kod pacijenata sa rizikom od toksičnosti metotreksata	Serb	03 Feb 2003	Voraxaze - EU/1/21/1586 - 13 Jan 2022
EU/3/12/1094	Modified recombinant human C-type natriuretic peptide	Lečenje ahondroplazije	BioMarin International Limited	24 Jan 2013	Voxzogo - EU/1/21/1577 - 01 Sep 2021
EU/3/17/1946	(2S,4R)-1-(2-(3-acetyl-5-(2-methylpyrimidine-5-yl)-1H-indazol-1-yl)acetyl)-N-(6-bromopyridine-2-yl)-4-fluoropyrrolidine-2-carboxamide	Lečenje paroksizmalne noćne hemoglobinurije	Alexion Europe SAS	12 Dec 2017	Voydeya - EU/1/24/1792 - 25 Apr 2024

EU/3/18/2012	Genetically modified replication-incompetent herpes simplex virus-1 expressing collagen VII	Lečenje bulozne epidermolize	Krystal Biotech Netherlands B.V.	16 Apr 2018	Vyjuvek - EU/1/25/1918 - 24 Apr 2025
EU/3/10/803	Chimeric monoclonal antibody against claudin-18 splice variant 2	Lečenje karcinoma želuca	Astellas Pharma Europe B.V.	26 Nov 2010	Vyloy - EU/1/24/1856 - 25 Sep 2024
EU/3/12/1066	tafamidis	Lečenje senilne sistemske amiloidoze	Pfizer Europe MA EEIG	08 Nov 2012	Vyndaqel - EU/1/11/717 - 19 Feb 2020
EU/3/18/1992	Efgartigimod alfa	Lečenje mijastenije gravis	Argenx	21 Mar 2018	Vyvgart - EU/1/22/1674 - 11 Aug 2022
EU/3/21/2555	Efgartigimod alfa	Lečenje hronične inflamatorne demijelinizacione polineuropatije	Argenx	14 Jan 2022	Vyvgart - EU/1/22/1674 - 20 Jun 2025
EU/3/11/942	Liposomal combination of cytarabine and daunorubicin	Lečenje akutne mijeloidne leukemije	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	Lečenje narkolepsije	Bioprojet Pharma	10 Jul 2007	Wakix - EU/1/15/1068 - 04 Apr 2016
EU/3/12/998	Autologous CD34+ cells transfected with lentiviral vector containing the Wiskott-Aldrich syndrome protein gene	Lečenje Viskot-Oldričovog sindroma	Fondazione Telethon Ets	06 Jun 2012	Waskyra - EU/1/25/1996 - 12 Jan 2026
EU/3/14/1249	Phosphorothioate oligonucleotide targeted to apolipoprotein C-III	Lečenje sindroma porodične hilomikronemije	Akcea Therapeutics Ireland Limited	19 Feb 2014	Waylivra - EU/1/19/1360 - 08 May 2019
EU/3/20/2278	Rilzabrutinib	Lečenje imunske trombocitopenije	Sanofi B.V.	04 Jun 2020	WAYRILZ - EU/1/25/1974 - 23 Dec 2025
EU/3/20/2369	Sotatercept	Lečenje plućne arterijske hipertenzije	Merck Sharp & Dohme B.V.	09 Dec 2020	Winrevair - EU/1/24/1850 - 27 Aug 2024
EU/3/01/056	Recombinant human acid sphingomyelinase	Lečenje Nimann-Pikove bolesti	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	Lečenje karcinoidnog sindroma	Serb	08 Oct 2009	Xermelo - EU/1/17/1224 - 20 Sep 2017

EU/3/19/2183	Mavorixafor	Lečenje WHIM sindroma	Norgine B.V.	25 Jul 2019	Xolremdi - EU/1/26/2017 - 28 Apr 2026
EU/3/17/1961	Gilteritinib	Lečenje akutne mijeloidne leukemije	Astellas Pharma Europe B.V.	17 Jan 2018	Xospata - EU/1/19/1399 - 28 Oct 2019
EU/3/14/1393	Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3 zeta chimeric antigen receptor	Lečenje difuznog B-krupnoćelijskog limfoma	Kite Pharma EU B.V.	16 Dec 2014	Yescarta - EU/1/18/1299 - 27 Aug 2018
EU/3/15/1553	Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor	Lečenje primarnog medijastinalnog B-krupnoćelijskog limfoma	Kite Pharma EU B.V.	09 Oct 2015	Yescarta - EU/1/18/1299 - 27 Aug 2018
EU/3/15/1579	Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor	Lečenje folikularnog limfoma	Kite Pharma EU B.V.	11 Nov 2015	Yescarta - EU/1/18/1299 - 23 Jun 2022
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)	Lečenje hipoparatireoidizma	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	Lečenje karcinoma jajnika	GlaxoSmithKline Trading Services Limited	04 Aug 2010	Zejula - EU/1/17/1235 - 20 Nov 2017
EU/3/20/2271	Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R, 4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate	Lečenje pri transplantaciji hematopoetskih matičnih ćelija	Cordex Biologics International Limited	22 Apr 2020	Zemcelpro - EU/1/25/1960 - 26 Aug 2025
EU/3/19/2143	Lurbinectedin	Lečenje mikrocelularnog karcinoma pluća	Pharma Mar S.A.	26 Feb 2019	ZEPZELCA - EU/1/26/2032 - 01 Jun 2026
EU/3/21/2458	Zanidatamab	Lečenje karcinoma bilijarnog trakta	Jazz Pharmaceuticals Ireland Limited	19 Jul 2021	Ziihera - EU/1/25/1931 - 30 Jun 2025
EU/3/18/2118	Lonafarnib	Lečenje Hačinson-Gilfordovog sindroma progerije	Integral Pharma Solutions EU 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	Lečenje spinalne mišićne atrofije	Novartis Europharm Limited	19 Jun 2015	Zolgensma - EU/1/20/1443 - 18 May 2020
EU/3/19/2224	Ganaxolone	Lečenje poremećaja usled nedostatka CDKL5	Immedica Pharma AB	13 Nov 2019	ZTALMY - EU/1/23/1743 - 28 Jul 2023
EU/3/20/2343	Retifanlimab	Lečenje karcinoma anusa	Incyte Biosciences Distribution B.V.	19 Oct 2020	ZYNYZ - EU/1/24/1800 - 06 Mar 2026
EU/3/22/2743	Retifanlimab	Lečenje karcinoma Merkelovih ćelija	Incyte Biosciences Distribution B.V.	13 Jan 2023	ZYNYZ - EU/1/24/1800 - 24 Apr 2024

Available from: European Commission. Community Register of orphan medicinal products [Internet]. Brussels: European Commission; 2026 [updated 2026 Nov 16; cited 2026 Nov 18]. Available from: https://ec.europa.eu/health/documents/community-register/html/reg_od_act.htm?sort=a