

# **Analýza dopadu zavedenia EMA Orphan Medicinal Product designation na úhrady liekov na zriedkavé ochorenia v Slovenskej republike v rokoch 2022-2025**

Bratislava, 17.12.2021

## Analýza dopadu zavedenia EMA Orphan Medicinal Product designation na úhrady liekov na zriedkavé ochorenia v Slovenskej republike v rokoch 2022-2025

### Metodika

#### 1. Priblíženie % podielu EMA OMP liekov európskym trhom

Zavádzame predpoklad, že v roku 2025 bude podiel EMA OMP liekov na úrovni 8,3%. Dopad na rozpočet je porovnávaný s konzervatívnym odhadom, v ktorom modelujeme 5% medziročný očakávaný nárast trhu s liekmi na zriedkavé ochorenia.

Pri kalkulácii výšky podielu EMA OMP vychádzame z celkového trhu A + AS úhrad, kde už rátame aj dopadom novelizácie 363/2011.

Následne korigujeme náklady na OMP lieky o odhadovanú 10% zľavu z MEA dohód. Tabuľka 2.

Zavádzame tiež korekciu 10% poníženia nákladov v dôsledku dodatočného odrátania úhrad zdravotných poisťovní na výnimkové lieky na zriedkavé ochorenia, kde predpokladáme zaradenie do kategorizačného zoznamu.

#### 2. Kontrolný model zavedenia liekov na zriedkavé ochorenia pre roky 2022 a 2023 na základe mapovania rozdielov medzi dostupnosťou na Slovensku a v štátoch CZ, HU a Lotyšsko

Pre mapovanie okolitých krajín sme dohľadali ceny cez EMA kód v zoznamoch jednotlivých krajín. Náklady na liečbu sú počítané ako 12x násobok priemeru z dohľadaných dostupných cien.

Pre liek Sirturo a Zolgensma sme nepočítali s liečenými pacientami v rámci kategorizovaných liekov.

Pri roku 2022 zvažujeme, že prvé vstupy sa môžu objaviť až v mesiaci august 2022.

Dopad zavedenia týchto konkrétnych liekov pri počítaní so 40% zľavou získanou cez vstupy s MEA dohodami odhadujeme dodatočné náklady v roku vo výške 32.6 miliónov eur.

Uvedený prepočet predstavuje veľmi hrubé zjednodušenie, nakoľko neberieme do úvahy reálne dĺžky liečby z SmPC a pri všetkých liekoch počítame s celoročným trvaním liečby počas 12 mesiacov.

Taktiež neberieme do úvahy nábeh pacientov na liečbu v rámci jednotlivých rokov, v ktorých sú iné náklady na pacienta zaradeného začiatkom roka a iné na konci roka.

## Výsledky

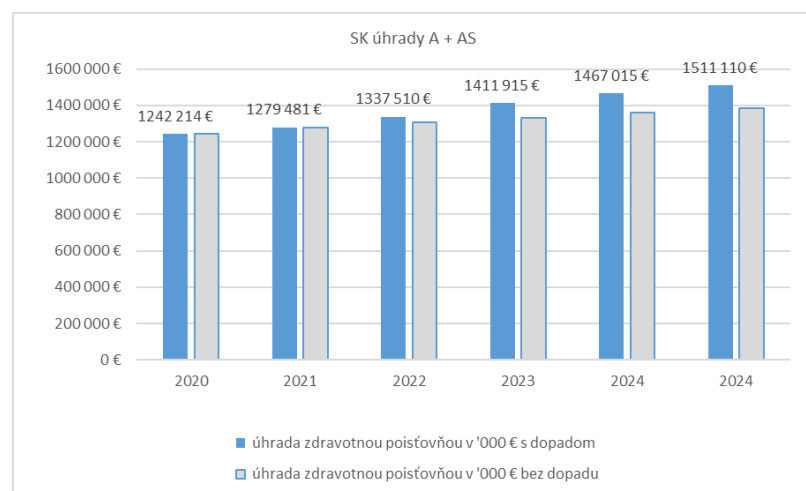
### 1. Príbliženie % podielu EMA OMP liekov európskym trhom a odhad veľkosti trhu s OMP a očakávaného dopadu na rozpočet

Ako základ pre odhad dopadu novelizácie 363/2011 je objem úhrad liekov A + AS konzervatívne modelovaný s medziročným nárastom 2%. Dopad novely 363/2011 je pridaný do porovnania s konzervatívnym modelom. Pre účely odhadovania dopadu zavedenia EMA OMP klasifikácie liekov pre zriedkavé ochorenia používame kalkuláciu s dopadom novely 363/2011.

#### Celý trh Slovensko

Spôsob úhrady / typ výdaja A + AS

	2020	2021	2022	2023	2024	2024
Medziročný nárast			2,0%	2,0%	2,0%	2,0%
Dopad 363			32 510 €	80 915 €	109 015 €	126 110 €
úhrada zdravotnou poisťovňou v '000 € s dopadom	1 242 214 €	1 279 481 €	1 337 510 €	1 411 915 €	1 467 015 €	1 511 110 €
úhrada zdravotnou poisťovňou v '000 € bez dopadu	1 242 214 €	1 279 481 €	1 305 000 €	1 331 000 €	1 358 000 €	1 385 000 €

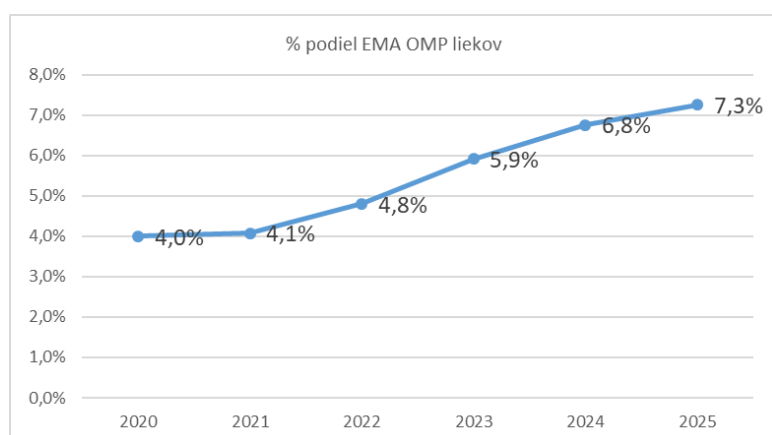


Tabuľka 1.

**Očakávaný dopad zavedenia EMA OMP**

Zavedenie OMP liekov s významným klinickým prínosom

	2020	2021	2022	2023	2024	2025
EMA OMP % podiel na celkových výdavkoch	4,0%	4,2%	4,8%	7,3%	8,3%	9,0%
EMA OMP v '000 € so súčasnou hranicou 1:50 000, 5%	49 689 €	52 173 €	54 782 €	57 521 €	60 397 €	63 417 €
EMA OMP v '000 € so zavedením OMP	49 689 €	52 173 €	64 200 €	103 070 €	122 349 €	135 395 €
Očakávaný dopad			9 420 €	45 550 €	61 950 €	71 980 €



Tabuľka 2.

**Očakávaný dopad zavedenie EMA OMP**

Dodatocné odrátanie priemernej zľavy od 2023 vo výš 10%

	2020	2021	2022	2023	2024	2025
EMA OMP % podiel na celkových výdavkoch	4,0%	4,2%	4,8%	7,3%	8,3%	9,0%
EMA OMP v '000 € so súčasnou hranicou 1:50 000, 5%	49 689 €	52 173 €	54 782 €	57 521 €	60 397 €	63 417 €
EMA OMP v '000 € so zavedením OMP	49 689 €	52 173 €	64 200 €	92 763 €	110 114 €	121 856 €
Očakávaný dopad			9 420 €	35 240 €	49 720 €	58 440 €

Tabuľka 3.

**Očakávaný dopad zavedenie EMA OMP**

Dodatocné odrátanie výnimkových liekov / od 2023 10%

	2020	2021	2022	2023	2024	2025
EMA OMP % podiel na celkových výdavkoch	4,0%	4,1%	4,8%	5,9%	6,8%	7,3%
EMA OMP v '000 € so súčasnou hranicou 1:50 000, 5%	49 689 €	52 173 €	54 782 €	57 521 €	60 397 €	63 417 €
EMA OMP v '000 € so zavedením OMP	49 689 €	52 173 €	64 200 €	83 487 €	99 103 €	109 670 €
Očakávaný dopad			9 420 €	25 970 €	38 710 €	46 250 €



Prehľad EMA OMP liekov so stanovenou úhradou aspoň na jednu indikáciu na Slovensku

EU #	Product	Indication	Tradename - EU product # - Implemented on	EU product #	dostupne na SK (ZKL 12/2021)
EU/3/11/939	Brentuximab vedotin	Treatment of cutaneous T-cell lymphoma	ADCETRIS - EU/1/12/794 - 19 Dec	EU/1/12/794	EU/1/12/794
EU/3/08/595	Monoclonal antibody against human	Treatment of peripheral T-cell lymphoma	ADCETRIS - EU/1/12/794 - 30 Oct 2012	EU/1/12/794	EU/1/12/794
EU/3/08/596	Monoclonal antibody against human	Treatment of Hodgkin lymphoma	ADCETRIS - EU/1/12/794 - 30 Oct 2012	EU/1/12/794	EU/1/12/794
EU/3/07/518	Methyl 4,6-diamino-2-[1-(2-	Treatment of pulmonary arterial hypertension	Adempas - EU/1/13/907 - 31 Mar	EU/1/13/907	EU/1/13/907
EU/3/09/667	Expanded human allogeneic	Treatment of anal fistula	Alofisel - EU/1/17/1261 - 27 Mar 2018	EU/1/17/1261	EU/1/17/1261
EU/3/07/453	Recombinant fusion protein	Treatment of haemophilia B (congenital factor IX	ALPROLIX - EU/1/16/1098 - 13 May	EU/1/16/1098	EU/1/16/1098
EU/3/13/1127	Inotuzumab ozogamicin	Treatment of B-cell acute lymphoblastic	Besponsa - EU/1/17/1200 - 03 Jul	EU/1/17/1200	EU/1/17/1200
EU/3/09/650	Blinatumomab	Treatment of acute lymphoblastic leukaemia	BLINCYTO - EU/1/15/1047 - 25 Nov	EU/1/15/1047	EU/1/15/1047
EU/3/09/629	Nanobody directed towards the	Treatment of thrombotic thrombocytopenic	Cablivi - EU/1/18/1305 - 04 Sep 2018	EU/1/18/1305	EU/1/18/1305
EU/3/07/514	(1R, 2R)-Octanoic acid [2-(2',3'-	Treatment of Gaucher Disease	Cerdeiga - EU/1/14/974 - 21 Jan 2015	EU/1/14/974	EU/1/14/974
EU/3/08/610	Cyclopropane-1,1-dicarboxylic acid [4-	Treatment of medullary thyroid carcinoma	Cometriq - EU/1/13/890 - 26 Mar	EU/1/13/890	EU/1/13/890
EU/3/14/1284	Isavuconazonium sulfate	Treatment of invasive aspergillosis	Cresemba - EU/1/15/1036 - 19 Oct	EU/1/15/1036	EU/1/15/1036
EU/3/14/1276	Isavuconazonium sulfate	Treatment of mucormycosis	Cresemba - EU/1/15/1036 - 19 Oct	EU/1/15/1036	EU/1/15/1036
EU/3/14/1351	Recombinant human monoclonal IgG1	Treatment of X-linked hypophosphataemia	CRYSVITA - EU/1/17/1262 - 21 Feb	EU/1/17/1262	EU/1/17/1262
EU/3/08/578	Cysteamine hydrochloride	Treatment of cystinosis	Cystadrops - EU/1/15/1049 - 23 Jan	EU/1/15/1049	EU/1/15/1049
EU/3/18/2020	Daratumumab	Treatment of AL amyloidosis	Darzalex - EU/1/16/1101 - 23 Jun	EU/1/16/1101	EU/1/16/1101
EU/3/13/1153	Daratumumab	Treatment of plasma cell myeloma	Darzalex - EU/1/16/1101 - 24 May	EU/1/16/1101	EU/1/16/1101
EU/3/06/368	1-deoxygalactonojirimycin	Treatment of Fabry disease	Galafold - EU/1/15/1082 - 31 May	EU/1/15/1082	EU/1/15/1082
EU/3/15/1504	Obinutuzumab	Treatment of follicular lymphoma	Gazyvaro - EU/1/14/937 - 15 Jun 2016	EU/1/14/937	EU/1/14/937
EU/3/12/1054	Obinutuzumab	Treatment of chronic lymphocytic leukaemia	Gazyvaro - EU/1/14/937 - 24 Jul 2014	EU/1/14/937	EU/1/14/937
EU/3/09/715	Benzamide, 3-(2-imidazo[1,2-	Treatment of acute lymphoblastic leukaemia	Iclusig - EU/1/13/839 - 03 Jul 2013	EU/1/13/839	EU/1/13/839
EU/3/09/716	Benzamide, 3-(2-imidazo[1,2-	Treatment of chronic myeloid leukaemia	Iclusig - EU/1/13/839 - 03 Jul 2013	EU/1/13/839	EU/1/13/839
EU/3/09/723	Recombinant fusion protein linking	Treatment of haemophilia B	IDELVION - EU/1/16/1095 - 13 May	EU/1/16/1095	EU/1/16/1095
EU/3/09/672	Pomalidomide	Treatment of multiple myeloma	Imnovid - EU/1/13/850 - 08 Aug 2013	EU/1/13/850	EU/1/13/850
EU/3/18/2116	Ivacaftor, N-(1,3-dimethyl-1H-	Treatment of cystic fibrosis	Kaftrio - EU/1/20/1468 - 21 Aug 2020	EU/1/20/1468	EU/1/20/1468
EU/3/08/556	N-(2,4-Di-tert-butyl-5-	Treatment of cystic fibrosis	Kalydeco - EU/1/12/782 - 25 Jul 2012	EU/1/12/782	EU/1/12/782
EU/3/13/1199	Sorafenib tosylate	Treatment of follicular thyroid cancer	Nexavar - EU/1/06/342 - 27 May 2014	EU/1/06/342	EU/1/06/342
EU/3/13/1200	Sorafenib tosylate	Treatment of papillary thyroid cancer	Nexavar - EU/1/06/342 - 27 May 2014	EU/1/06/342	EU/1/06/342
EU/3/11/899	2,2'-(2-((1R)-1-(((2,5-	Treatment of multiple myeloma	Ninlaro - EU/1/16/1094 - 23 Nov 2016	EU/1/16/1094	EU/1/16/1094
EU/3/11/909	Macitentan	Treatment of pulmonary arterial hypertension	Opsumit - EU/1/13/893 - 27 Dec 2013	EU/1/13/893	EU/1/13/893
EU/3/10/733	Glyceryl tri-(4-phenylbutyrate)	Treatment of carbamoyl-phosphate synthase-1	Ravicti - EU/1/15/1062 - 01 Dec 2015	EU/1/15/1062	EU/1/15/1062
EU/3/10/734	Glyceryl tri-(4-phenylbutyrate)	Treatment of ornithine carbamoyltransferase	Ravicti - EU/1/15/1062 - 01 Dec 2015	EU/1/15/1062	EU/1/15/1062
EU/3/10/735	Glyceryl tri-(4-phenylbutyrate)	Treatment of citrullinaemia type 1	Ravicti - EU/1/15/1062 - 01 Dec 2015	EU/1/15/1062	EU/1/15/1062
EU/3/10/736	Glyceryl tri-(4-phenylbutyrate)	Treatment of argininosuccinic aciduria	Ravicti - EU/1/15/1062 - 01 Dec 2015	EU/1/15/1062	EU/1/15/1062
EU/3/10/737	Glyceryl tri-(4-phenylbutyrate)	Treatment of hyperargininaemia	Ravicti - EU/1/15/1062 - 01 Dec 2015	EU/1/15/1062	EU/1/15/1062
EU/3/10/738	Glyceryl tri-(4-phenylbutyrate)	Treatment of ornithine translocase deficiency	Ravicti - EU/1/15/1062 - 01 Dec 2015	EU/1/15/1062	EU/1/15/1062
EU/3/01/077	[gly2] Recombinant human glucagon-	Treatment of Short Bowel Syndrome	Revestive - EU/1/12/787 - 04 Sep	EU/1/12/787	EU/1/12/787
EU/3/10/765	Midostaurin	Treatment of mastocytosis	Rydapt - EU/1/17/1218 - 20 Sep 2017	EU/1/17/1218	EU/1/17/1218
EU/3/04/214	Midostaurin	Treatment of acute myeloid leukaemia	Rydapt - EU/1/17/1218 - 20 Sep 2017	EU/1/17/1218	EU/1/17/1218
EU/3/09/670	pasireotide	Treatment of acromegaly	Signifor - EU/1/12/753 - 21 Nov 2014	EU/1/12/753	EU/1/12/753
EU/3/09/671	pasireotide	Treatment of Cushing's disease	Signifor - EU/1/12/753 - 27 Apr 2012	EU/1/12/753	EU/1/12/753
EU/3/12/976	Antisense oligonucleotide targeted	Treatment of Sq spinal muscular atrophy	Spinraza - EU/1/17/1188 - 01 Jun 2017	EU/1/17/1188	EU/1/17/1188
EU/3/15/1551	Recombinant human IgG1 kappa light	Treatment of hereditary angioedema	TAKHZYRO - EU/1/18/1340 - 26 Nov	EU/1/18/1340	EU/1/18/1340
EU/3/03/140	Tobramycin (inhalation powder)	Treatment of Pseudomonas aeruginosa lung	TOBI Podhaler - EU/1/10/652 - 25 Jul	EU/1/10/652	EU/1/10/652
EU/3/05/278	(3-[5-(2-fluoro-phenyl)]-	Treatment of Duchenne muscular dystrophy	Translarna - EU/1/13/902 - 05 Aug	EU/1/13/902	EU/1/13/902
EU/3/10/752	Velaglucerase alfa	Treatment of Gaucher disease	VPRIV - EU/1/10/646 - 30 Aug 2010	EU/1/10/646	EU/1/10/646
EU/3/09/628	Mercaptopurine (oral suspension)	Treatment of acute lymphoblastic leukaemia	Xaluprine - EU/1/11/727 - 13 Mar	EU/1/11/727	EU/1/11/727
EU/3/09/661	(S)-ethyl 2-amino-3-(4-(2-amino-6-	Treatment of carcinoid syndrome	Xermelo - EU/1/17/1224 - 20 Sep	EU/1/17/1224	EU/1/17/1224

**Prehľad EMA OMP liekov bez stanovenej úhrady na Slovensku**

Product	Indication	Tradename - EU product # - Implemented on	EU product #
Autologous T lymphocyte-enriched	Treatment of multiple myeloma	Abecma - EU/1/21/1539 - 19 Aug	EU/1/21/1539
Humanised monoclonal antibody	Treatment of sickle cell disease	Adakveo - EU/1/20/1476 - 29 Oct	EU/1/20/1476
Glibenclamide	Treatment of neonatal diabetes	AMGLIDIA - EU/1/18/1279 - 28 May	EU/1/18/1279
Amikacin sulfate	Treatment of nontuberculous mycobacterial lung	ARIKAYCE liposomal - EU/1/20/1469 -	EU/1/20/1469
(S)-1-(4-fluorophenyl)-1-(2-(4-(6-(1-	Treatment of gastrointestinal stromal tumours	AYVAKYT - EU/1/20/1473 - 25 Sep	EU/1/20/1473
Humanised monoclonal antibody	Treatment of multiple myeloma	BLENREP - EU/1/20/1474 - 26 Aug	EU/1/20/1474
Recombinant human tripeptidyl-	Treatment of neuronal ceroid lipofuscinosis type	Brineura - EU/1/17/1192 - 01 Jun	EU/1/17/1192
Mannitolol	Treatment of cystic fibrosis	Bronchitol - EU/1/12/760 - 18 Apr	EU/1/12/760
(2S)-2-(((2R)-2-(((3,3-dibutyl-7-	Treatment of progressive familial intrahepatic	Bylvay - EU/1/21/1566 - 19 Jul 2021	EU/1/21/1566
Human coagulation factor X	Treatment of hereditary factor X deficiency	Coagadex - EU/1/16/1087 - 18 Mar	EU/1/16/1087
Decitabine	Treatment of acute myeloid leukaemia	Dacogen - EU/1/12/792 - 24 Sep 2012	EU/1/12/792
Glasdegib maleate	Treatment of acute myeloid leukaemia	Daurismo - EU/1/20/1451 - 29 Jun	EU/1/20/1451
Defibrotide	Treatment of hepatic veno-occlusive disease	Defitelio - EU/1/13/878 - 22 Oct 2013	EU/1/13/878
(R)-2-Methyl-6-nitro-2-[4-[4-(4-	Treatment of tuberculosis	Deltyba - EU/1/13/875 - 30 Apr 2014	EU/1/13/875
(S)-2-nitro-6-(4-	Treatment of tuberculosis	Dovprela - EU/1/20/1437 - 04 Aug	EU/1/20/1437
Recombinant human interleukin-3	Treatment of blastic plasmacytoid dendritic cell	ELZONRIS - EU/1/20/1504 - 13 Jan	EU/1/20/1504
Humanised anti-IL-6 receptor	Treatment of neuromyelitis optica spectrum	Enspryng - EU/1/21/1559 - 29 Jun	EU/1/21/1559
Cannabidiol	Treatment of tuberous sclerosis	Epidyolex - EU/1/19/1389 - 20 Apr	EU/1/19/1389
Cannabidiol	Treatment of Lennox-Gastaut syndrome	Epidyolex - EU/1/19/1389 - 23 Sep	EU/1/19/1389
Cannabidiol	Treatment of Dravet syndrome	Epidyolex - EU/1/19/1389 - 23 Sep	EU/1/19/1389
Risdiplam	Treatment of spinal muscular atrophy	Evrysdi - EU/1/21/1531 - 29 Mar 2021	EU/1/21/1531
Panobinostat	Treatment of multiple myeloma	Farydak - EU/1/15/1023 - 01 Sep 2015	EU/1/15/1023
Fenfluramine hydrochloride	Treatment of Dravet syndrome	Fintepla - EU/1/20/1491 - 21 Dec	EU/1/20/1491
Synthetic double-stranded siRNA	Treatment of acute hepatic porphyria	Givlaari - EU/1/20/1428 - 04 Mar 2020	EU/1/20/1428
Para-aminosalicylic acid	Treatment of tuberculosis	Granupas - EU/1/13/896 - 09 Apr 2014	EU/1/13/896
Synthetic 47-amino-acid N-	Treatment of hepatitis delta virus infection	Hepcludex - EU/1/20/1446 - 05 Aug	EU/1/20/1446
Tasimelteon	Treatment of non-24-hour sleep-wake disorders	Hetlioz - EU/1/15/1008 - 07 Jul 2015	EU/1/15/1008
Ex-vivo expanded autologous human	Treatment of corneal lesions, with associated	Holoclar - EU/1/14/987 - 19 Feb 2015	EU/1/14/987
Chenodeoxycholic acid	Treatment of inborn errors of primary bile acid	Chenodeoxycholic acid Leadiant -	EU/1/16/1110
Recombinant IgG degrading enzyme	Prevention of graft rejection following solid	Idefirix - EU/1/20/1471 - 01 Sep 2020	EU/1/20/1471
Setmelanotide	Treatment of leptin receptor deficiency	Imcivree - EU/1/21/1564 - 19 Jul 2021	EU/1/21/1564
Setmelanotide	Treatment of pro-opiomelanocortin deficiency	Imcivree - EU/1/21/1564 - 19 Jul 2021	EU/1/21/1564
N-tert-butyl-3-[[5-methyl-2-[[4-(2-	Treatment of post-essential thrombocythaemia	Inrebic - EU/1/20/1514 - 09 Feb 2021	EU/1/20/1514
N-tert-butyl-3-[[5-methyl-2-[[4-(2-	Treatment of post-polycythaemia vera	Inrebic - EU/1/20/1514 - 09 Feb 2021	EU/1/20/1514
N-tert-butyl-3-[[5-methyl-2-[[4-(2-	Treatment of primary myelofibrosis	Inrebic - EU/1/20/1514 - 09 Feb 2021	EU/1/20/1514
Osilodrostat	Treatment of Cushing's syndrome	Isturisa - EU/1/19/1407 - 13 Jan 2020	EU/1/19/1407
Budesonide	Treatment of eosinophilic oesophagitis	Jorveza - EU/1/17/1254 - 10 Jan 2018	EU/1/17/1254
Recombinant human lysosomal acid	Treatment of lysosomal acid lipase deficiency	Kanuma - EU/1/15/1033 - 01 Sep 2015	EU/1/15/1033
Ketoconazole	Treatment of Cushing's syndrome	Ketoconazole HRA - EU/1/14/965 - 21	EU/1/14/965
Selumetinib	Treatment of neurofibromatosis type 1	Koselugo - EU/1/21/1552 - 19 Jun	EU/1/21/1552
Autologous T cells transduced with	Treatment of diffuse large B-cell lymphoma	Kymriah - EU/1/18/1297 - 27 Aug	EU/1/18/1297
Autologous T cells transduced with	Treatment of B-lymphoblastic	Kymriah - EU/1/18/1297 - 27 Aug	EU/1/18/1297
Carfilzomib	Treatment of multiple myeloma	Kyprolis - EU/1/15/1060 - 23 Nov	EU/1/15/1060
Recombinant human $\alpha$ -Mannosidase	Treatment of $\alpha$ -Mannosidosis	Lamzedo - EU/1/17/1258 - 27 Mar	EU/1/17/1258
Chlormethine	Treatment of cutaneous T-cell lymphoma	Ledaga - EU/1/16/1171 - 07 Mar 2017	EU/1/16/1171
Autologous CD34+ cells transfected	Treatment of metachromatic leukodystrophy	Libmeldy - EU/1/20/1493 - 18 Dec	EU/1/20/1493
Lutetium (177Lu)-N-[(4,7,10-	Treatment of gastro-entero-pancreatic	Lutathera - EU/1/17/1226 - 28 Sep	EU/1/17/1226
Adenovirus-associated viral vector	Treatment of inherited retinal dystrophies	Luxturna - EU/1/18/1331 - 05 Dec	EU/1/18/1331
Adenovirus associated viral vector	Treatment of inherited retinal dystrophies	Luxturna - EU/1/18/1331 - 05 Dec	EU/1/18/1331
Recombinant human beta-	Treatment of mucopolysaccharidosis type VII (Sly)	Mepsevii - EU/1/18/1301 - 27 Aug	EU/1/18/1301
Humanised Fc engineered	Treatment of diffuse large B-cell lymphoma	Minjuvi - EU/1/21/1570 - 01 Sep 2021	EU/1/21/1570
Metreleptin	Treatment of Familial Partial Lipodystrophy	Myalepta - EU/1/18/1276 - 01 Aug	EU/1/18/1276
Metreleptin	Treatment of Barraquer-Simons syndrome	Myalepta - EU/1/18/1276 - 01 Aug	EU/1/18/1276
Metreleptin	Treatment of Lawrence syndrome	Myalepta - EU/1/18/1276 - 01 Aug	EU/1/18/1276
Metreleptin	Treatment of Berardinelli-Seip syndrome	Myalepta - EU/1/18/1276 - 01 Aug	EU/1/18/1276

**Prehľad EMA OMP liekov bez stanovenej úhrady na Slovensku, pokračovanie**

Product	Indication	Tradename - EU product # - Implemented on	EU product #
Gemtuzumab Ozogamicin	Treatment of acute myeloid leukaemia	Mylotarg - EU/1/18/1277 - 23 Apr	EU/1/18/1277
Mexiletine hydrochloride	Treatment of myotonic disorders	Namuscla - EU/1/18/1325 - 20 Dec	EU/1/18/1325
Recombinant human parathyroid	Treatment of hypoparathyroidism	Natpar - EU/1/15/1078 - 26 Apr 2017	EU/1/15/1078
Purified bromelain	Treatment of partial deep dermal and full	NexoBrid - EU/1/12/803 - 20 Dec	EU/1/12/803
Obiltoximab	Treatment of anthrax	Obiltoximab SFL - EU/1/20/1485 -	EU/1/20/1485
6alpha-ethyl-chenodeoxycholic acid	Treatment of primary biliary cirrhosis	Ocaliva - EU/1/16/1139 - 15 Dec 2016	EU/1/16/1139
Nanoliposomal irinotecan	Treatment of pancreatic cancer	Onivyde pegylated liposomal -	EU/1/16/1130
Synthetic double-stranded siRNA	Treatment of transthyretin-mediated	Onpattro - EU/1/18/1320 - 29 Aug	EU/1/18/1320
Cholic acid	Treatment of inborn errors in primary bile acid	Orphacol - EU/1/13/870 - 16 Sep 2013	EU/1/13/870
Recombinant human nerve growth	Treatment of neurotrophic keratitis	OXERVATE - EU/1/17/1197 - 10 Jul	EU/1/17/1197
Synthetic double-stranded siRNA	Treatment of primary hyperoxaluria	Oxlumo - EU/1/20/1496 - 23 Nov	EU/1/20/1496
Pegylated recombinant	Treatment of hyperphenylalaninaemia	Palynziq - EU/1/19/1362 - 08 May	EU/1/19/1362
Pemigatinib	Treatment of biliary tract cancer	Pemazyre - EU/1/21/1535 - 29 Mar	EU/1/21/1535
Hydrocortisone (modified release	Treatment of adrenal insufficiency	Plenadren - EU/1/11/715 - 14 Nov	EU/1/11/715
Polatuzumab vedotin	Treatment of diffuse large B-cell lymphoma	Polivy - EU/1/19/1388 - 20 Jan 2020	EU/1/19/1388
Mogamulizumab	Treatment of cutaneous T-cell lymphoma	Poteligeo - EU/1/18/1335 - 26 Nov	EU/1/18/1335
(S)-{8-fluoro-2-2[4-(3- Cysteamine bitartrate	Prevention of cytomegalovirus disease in Treatment of cystinosis	PREVYMIS - EU/1/17/1245 - 10 Jan Procysbi - EU/1/13/861 - 10 Sep 2013	EU/1/17/1245 EU/1/13/861
Chimeric monoclonal antibody	Treatment of neuroblastoma	Qarziba - EU/1/17/1191 - 11 May 2017	EU/1/17/1191
Idebenone	Treatment of Leber's hereditary optic neuropathy	Raxone - EU/1/15/1020 - 10 Sep 2015	EU/1/15/1020
Recombinant fusion protein	Treatment of myelodysplastic syndromes	Reblozyl - EU/1/20/1452 - 26 Jun	EU/1/20/1452
Recombinant fusion protein	Treatment of beta-thalassaemia intermedia and	Reblozyl - EU/1/20/1452 - 26 Jun	EU/1/20/1452
[Nle4, D-Phe7]-alpha-melanocyte	Treatment of erythropoietic protoporphyria	SCENESSE - EU/1/14/969 - 29 Dec	EU/1/14/969
(1R,2S) 6-bromo-alpha-[2- Autologous haematopoietic stem	Treatment of tuberculosis Treatment of adrenoleukodystrophy	Sirturo - EU/1/13/901 - 07 Mar 2014 Skysona - EU/1/21/1563 - 19 Jul 2021	EU/1/13/901 EU/1/21/1563
Somapacitan	Treatment of growth hormone deficiency	Sogroya - EU/1/20/1501 - 06 Apr 2021	EU/1/20/1501
Eculizumab	Treatment of myasthenia gravis	Soliris - EU/1/07/393 - 17 Aug 2017	EU/1/07/393
Eculizumab	Treatment of neuromyelitis optica spectrum	Soliris - EU/1/07/393 - 28 Aug 2019	EU/1/07/393
Eculizumab	Treatment of atypical haemolytic uremic	Soliris - EU/1/07/393 - 29 Nov 2011	EU/1/07/393
Gallium (68Ga)-edotreotide	Diagnosis of gastro-entero-pancreatic	SomaKit TOC - EU/1/16/1141 - 12 Dec	EU/1/16/1141
Recombinant human tissue non- Autologous CD34+ cells transfected	Treatment of hypophosphatasia Treatment of severe combined	Strensiq - EU/1/15/1015 - 01 Sep Strimvelis - EU/1/16/1097 - 30 May	EU/1/15/1015 EU/1/16/1097
Chimeric-anti-interleukin-6	Treatment of Castleman's disease	Sylvant - EU/1/14/928 - 27 May 2014	EU/1/14/928
1-(2,2-difluoro-2H-1,3-benzodioxol-5- Autologous peripheral blood T cells	Treatment of cystic fibrosis Treatment of mantle cell lymphoma	Symkevi - EU/1/18/1306 - 06 Nov Tecartus - EU/1/20/1492 - 15 Dec	EU/1/18/1306 EU/1/20/1492
Phosphorothioate oligonucleotide	Treatment of ATTR amyloidosis	Tegsedi - EU/1/18/1296 - 10 Jul 2018	EU/1/18/1296
Treosulfan	Conditioning treatment prior to haematopoietic	Trecondi - EU/1/18/1351 - 24 Jun	EU/1/18/1351
Treprostinil sodium	Treatment of chronic thromboembolic pulmonary	Trepulmix - EU/1/19/1419 - 07 Apr	EU/1/19/1419
Ciclosporin	Treatment of vernal keratoconjunctivitis	Verkazia - EU/1/17/1219 - 10 Jul 2018	EU/1/17/1219
Recombinant human N- Everolimus	Treatment of mucopolysaccharidosis, type IVA Treatment of tuberous sclerosis	Vimizim - EU/1/14/914 - 30 Apr 2014 Votubia - EU/1/11/710 - 06 Sep 2011	EU/1/14/914 EU/1/11/710
Modified recombinant human C-type	Treatment of achondroplasia	Voxzogo - EU/1/21/1577 - 01 Sep	EU/1/21/1577
N-methyl D-(2,3,4,5,6-pentahydroxy- tafamidis	Treatment of familial amyloid polyneuropathy Treatment of senile systemic amyloidosis	Vyndaqel - EU/1/11/717 - 18 Nov Vyndaqel - EU/1/11/717 - 19 Feb	EU/1/11/717 EU/1/11/717
Liposomal combination of cytarabine	Treatment of acute myeloid leukaemia	Vyxeos liposomal - EU/1/18/1308 -	EU/1/18/1308
1-{3-[3-(4- Phosphorothioate oligonucleotide	Treatment of narcolepsy Treatment of familial chylomicronemia syndrome	Wakix - EU/1/15/1068 - 04 Apr 2016 Waylivra - EU/1/19/1360 - 08 May	EU/1/15/1068 EU/1/19/1360
Gilteritinib	Treatment of acute myeloid leukaemia	Xospata - EU/1/19/1399 - 28 Oct 2019	EU/1/19/1399
Autologous T cells transduced with	Treatment of primary mediastinal large B-cell	Yescarta - EU/1/18/1299 - 27 Aug	EU/1/18/1299
Autologous T cells transduced with	Treatment of diffuse large B cell lymphoma	Yescarta - EU/1/18/1299 - 27 Aug	EU/1/18/1299
(3S)-3-[4-[7-(aminocarbonyl)-2H- Adeno-associated viral vector	Treatment of ovarian cancer Treatment of spinal muscular atrophy	Zejula - EU/1/17/1235 - 20 Nov 2017 Zolgensma - EU/1/20/1443 - 18 May	EU/1/17/1235 EU/1/20/1443
Autologous CD34+ haematopoietic	Treatment of beta-thalassaemia intermedia and	Zynteglo - EU/1/19/1367 - 04 Jun	EU/1/19/1367

## 2. Kontrolný prepočet zavedenia liekov na zriedkavé ochorenia pre roky 2022 a 2023 na základe mapovania rozdielov medzi dostupnosťou na Slovensku a v štátoch CZ, HU a Lotyšsko

**EMA OMP lieky s dohľadanými cenami v okolitých štátoch Česká republika, Maďarsko a Lotyšsko, ktoré nemajú stanovenú úhradu na Slovensku**

EU #	Product	Indication	Prevalencia Orphanet	Eligible patients	CZK	Maďarsko	Lotyšsko
EU/3/05/325	Mannitolum	Treatment of cystic fibrosis	1	550	0	252	
EU/3/13/1181	Budesonide	Treatment of eosinophilic oesophagitis	5	2750			110
EU/3/12/965	Ketoconazole	Treatment of Cushing's syndrome	1,2	660			660
EU/3/00/005	Gemtuzumab Ozogamicin	Treatment of acute myeloid leukaemia	1,6	880	6 906		7 200
EU/3/11/933	Nanoliposomal irinotecan	Treatment of pancreatic cancer	1,4	770	801		840
EU/3/07/434	Idebenone	Treatment of Leber's hereditary optic neuropathy	1	550	3 911		
EU/3/05/314	(1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethanol	Treatment of tuberculosis	--				12 954
EU/3/10/764	Everolimus	Treatment of tuberous sclerosis	1	550			1 234
EU/3/17/1961	Gilteritinib	Treatment of acute myeloid leukaemia	0,4	220	16 456		
EU/3/10/760	(3S)-3-[4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl] piperidine tosylate monohydrate salt	Treatment of ovarian cancer	2,4	1320			5 018
EU/3/15/1509	Adeno-associated viral vector serotype 9 containing the human SMN gene	Treatment of spinal muscular atrophy					1 945 000



### Dopad modelovania príchodu na trh vybraných EMA OMP pre roky 2022 a 2025

Pre vybraných 10 diagnóz predpokladáme úhradu v krajinách s dohľadanými cenami, a podľa Foltánová et al., kde kolektív autorov odhaduje, že na Slovensku je liečených 10% vhodných pacientov s raritnými ochoreniami odhadujeme počet novoliečených pacientov v rozmedzí od 5% do 20% v rámci skupiny.

EU #	Product	Indication	Prevalenci a Orphanet	Eligible patients	Liečení pri vstupe 2022	Náklady 2022	Liečení 2025	Náklady 2025
EU/3/05/325	Mannitolum	Treatment of cystic fibrosis	1	550	50	15 773 €	110	991 829 €
EU/3/13/1181	Budesonide	Treatment of eosinophilic oesophagitis	5	2750	34	9 461 €	137,5	635 805 €
EU/3/12/965	Ketoconazole	Treatment of Cushing's syndrome	1,2	660	33	54 450 €	132	3 659 040 €
EU/3/00/005	Gemtuzumab Ozogamicin	Treatment of acute myeloid leukaemia	1,6	880	11	193 954 €	44	13 033 727 €
EU/3/11/933	Nanoliposomal irinotecan	Treatment of pancreatic cancer	1,4	770	39	78 968 €	154	5 306 642 €
EU/3/07/434	Idebenone	Treatment of Leber's hereditary optic neuropathy	1	550	7	67 224 €	27,5	4 517 437 €
EU/3/05/314	(1R,2S)-6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethanol	Treatment of tuberculosis	--			- €		- €
EU/3/10/764	Everolimus	Treatment of tuberous sclerosis	1	550	28	84 837 €	110	5 701 035 €
EU/3/17/1961	Gilteritinib	Treatment of acute myeloid leukaemia	0,4	220	6	226 271 €	22	15 205 432 €
EU/3/10/760	(3S)-3-(4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl) piperidine tosylate monohydrate salt	Treatment of ovarian cancer	2,4	1320	17	206 985 €	66	13 909 402 €
EU/3/15/1509	Adeno-associated viral vector serotype 9 containing the human SMN gene	Treatment of spinal muscular atrophy						

Dopad zavedenia týchto konkrétnych liekov pri počítaní so 40% zľavou získanou cez vstupy s MEA dohodami odhadujeme dodatočné náklady v roku vo výške 32.6 miliónov eur.

Uvedený prepočet predstavuje veľmi hrubé zjednodušenie, nakoľko neberieme do úvahy reálne dĺžky liečby z SmPC a pri všetkých liekoch počítame s celoročným trvaním liečby počas 12 mesiacov.

Taktiež neberieme do úvahy nábeh pacientov na liečbu v rámci jednotlivých rokov, v ktorých sú iné náklady na pacienta zaradeného začiatkom roka a iné na konci roka.

#### Dopad zavedenia EMA OMP / gap analýza CZ, HU, LT

Parameter	Hodnoty	
	2022	2025
Počet liečených pacientov	223	803
Očakávaný dopad nákladov na lieky	824 800 €	55 357 600 €
Očakávaný dopad nákladov na lieky pri zavedení 40% zľavy / MEA	485 409 €	32 619 500 €