# Orphan medicinal products with marketing authorisation

http://ec.europa.eu/health/documents/community-register/html/index_en.htm

**List of Orphan Medicinal Products with Marketing Authorisation (as of 30 April 2019)**

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| **2001** | | | | | |
| 1 | 1 | **Fabrazyme** (agalsidase beta) **EXPIRED** | Genzyme BV | Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency). | |
| 2 | 2 | **Replagal** (agalsidase alpha) **EXPIRED** | Shire Human Genetic Therapies AB | Long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency). | |
| 3 | 3 | **Glivec** (imatinib) **EXPIRED** | Novartis Europharm Limited | Treatment of adult and **paediatric** patients with newly diagnosed Philadelphia chromosome (bcrabl) positive (Ph+) chronic myeloid leukaemia (CML) for whom bone marrow transplantation is not considered as the first line of treatment. Glivec is also indicated for the treatment of adult and paediatric patients with Ph+ CML in chronic phase after failure of interferon-alpha therapy, or in accelerated phase or blast crisis. The effect of Glivec on the outcome of bone marrow transplantation has not been determined. | |
| 4 | | **Glivec** (imatinib) **WITHDRAWN** | Novartis Europharm Limited | Glivec is also indicated for the treatment of adult patients with Kit (CD 117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumours (GIST). | 2002 |
| 5 | | **Glivec** (imatinib) **WITHDRAWN** | Novartis Europharm Limited | Treatment of adult patients with unresectable recurrent and/or metastatic dermafibrosarcoma protuberans | 2006 |
| 6 | | **Glivec** (imatinib) **WITHDRAWN** | Novartis Europharm Limited | Treatment of adult patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukaemia (Ph+ ALL) as monotherapy | 2006 |
| 7 | | **Glivec** (imatinib) **WITHDRAWN** | Novartis Europharm Limited | Treatment of adult patients with myelodysplastic/ myeloproliferative diseases (MDS/MPD) associated with PDGFR gene re-arrangement | 2006 |
| 8 | | **Glivec** (imatinib) **WITHDRAWN** | Novartis Europharm Limited | Treatment of adult patients with hypereosinophilic syndrome (HES) and chronic eosinophilic leukaemia (CEL) | 2006 |
| **2002** | | | | | |
| 9 | 4 | **Trisenox** (arsenic trioxide) **EXPIRED** | Cephalon Europe | "For induction of remission and consolidation in adult patients with relapsed/refractory acute promyelocytic leukaemia (APL), characterised by the presence of the t(15;17) translocation and/or the presence of the Pro-Myelocytic Leukaemia/Retinoic-Acid-Receptor-alpha (PML/RAR-alpha) gene. Previous treatment | |

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| | | | | should have included a retinoid and chemotherapy.<br>The response rate of other acute myelogenous leukaemia subtypes to TRISENOX has not been examined." | |
| 10 | 5 | **Tracleer** (bosentan) **EXPIRED** | Actelion Registration Limited | "Treatment of pulmonary arterial hypertension (PAH) to improve exercise capacity and symptoms in patients with WHO functional class III.Efficacy has been shown in:<br>- Primary (idiopathic and familial) PAH.<br>- PAH secondary to scleroderma without significant interstitial pulmonary disease.<br>- PAH associated with congenital systemic-to-pulmonary shunts and Eisenmenger's physiology.<br>- Some improvements have also been shown in patients with PAH WHO functional class II." | |
| 11 | | **Tracleer** (bosentan) **WITHDRAWN** | Actelion Registration Limited | Indicated to reduce the number of new digital ulcers in patients with systemic sclerosis and ongoing digital ulcer disease. | 2007 |
| 12 | 6 | **Somavert** (pegvisomant) **EXPIRED** | Pfizer Limited | Treatment of patients with acromegaly who have had an inadequate response to surgery and/or radiation therapy and in whom an appropriate medical treatment with somatostatin analogues did not normalize IGF-I concentrations or was not tolerated. | |
| 13 | 7 | **Zavesca** (miglustat) **EXPIRED** | Actelion Registration Limited | Zavesca is indicated for the oral treatment of mild to moderate type 1 Gaucher disease. Zavesca may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable. | |
| 14 | | **Zavesca** (miglustat) **EXPIRED** | Actelion Registration Limited | Extension of Indication – to include the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease. | 2009 |
| **2003** | | | | | |
| 15 | 8 | **Carbaglu** (carglumic acid) **EXPIRED** | Orphan Europe Sarl | Treatment of hyperammonaemia due to N-acetylglutamate synthase deficiency. | |
| 16 | | **Carbaglu** (carglumic acid) | Orphan Europe SARL - France | This variation concerns an extension of indication of Carbaglu to add the treatment of hyperammoniemia due to isovaleric acidaemia, methylmalonic acidaemia and propionic acidaemia. 6/2021 | 2011 |
| 17 | 9 | **Aldurazyme** (laronidase) **EXPIRED** | Genzyme Europe BV | Aldurazyme is indicated for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis I (MPSI; a [alpha]-L-iduronidase deficiency) to treat the non-neurological manifestations of the disease | |
| 18 | 10 | **Busilvex** (busulfan) **EXPIRED** | Pierre Fabre Medicament | Busilvex followed by cyclophosphamide (BuCy2) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell transplantation (HPCT) in adult patients when the combination is considered the best available option.<br>Busilvex followed by cyclophosphamide (BuCy4) or melphalan (BuMel) is indicated as conditioning treatment prior to conventional haematopoietic progenitor cell | |

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| | | | | transplantation in **paediatric** patients. | |
| 19 | 11 | **Ventavis** (iloprost) **EXPIRED** | Schering AG | Treatment of patients with primary pulmonary hypertension, classified as NYHA functional class III, to improve exercise capacity and symptoms. | |
| 20 | 12 | **Onsenal** (celecoxib) **WITHDRAWN FROM THE MARKET - SAFETY** | Pharmacia-Pfizer EEIG | For the reduction of the number of adenomatous intestinal polyps in familial adenomatous polyposis (FAP), as an adjunct to surgery and further endoscopic surveillance. | |
| **2004** | | | | | |
| 21 | 13 | **Photobarr** (porfimer sodium) **WITHDRAWN FROM THE MARKET - SAFETY** | Axcan Pharma International BV | Photodynamic therapy (PDT) with porfimer sodium is indicated for ablation of high grade dysplasia (HGD) in patients with Barrett's Esophagus (BE) | |
| 22 | 14 | **Litak** (cladribine,B) **EXPIRED** | Lipomed GmbH | Treatment of hairy cell leukaemia | |
| 23 | 15 | **Lysodren** (mitotane) **EXPIRED** | Laboratoire HRA Pharma | Symptomatic treatment of advanced (unresectable, metastatic or relapsed) adrenal cortical carcinoma. The effect of Lysodren on non-functional adrenal cortical carcinoma is not established. | |
| 24 | 16 | **Pedea** (ibuprofen) **EXPIRED** | Orphan Europe SARL | Indicated to close a patent ductus arteriosus in preterm **newborn** infants | |
| 25 | 17 | **Wilzin** (zinc-acetate dihydrate) **EXPIRED** | Orphan Europe SARL | Treatment of Wilson's disease | |
| 26 | 18 | **Xagrid** (anegrelide hydrochloride) **EXPIRED** | Shire Pharmaceutic als Ltd | Reduction of elevated platelet counts in at risk essential thrombocythaemia patients who are intolerant to their current therapy or whose elevated platelet counts are not reduced to an acceptable level by their current therapy. | |
| **2005** | | | | | |
| 27 | 19 | **Prialt** (ziconotide) **EXPIRED** | Elan Pharma Int. | Treatment of chronic pain requiring intrathecal (IT) analgesia in patients who fail to obtain adequate analgesia and/or suffer intolerable adverse events with systemic opioids | |
| 28 | 20 | **Orfadin** (nitisinone) **EXPIRED** | Swedish Orphan Int. | Treatment of patients with confirmed diagnosis of hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine. | |
| 29 | 21 | **Xyrem** (sodium oxybate) **WITHDRAWN** | UCB Pharma Ltd | Treatment of narcolepsy with cataplexy in adult patients. | |

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| 30 | 22 | **Revatio** (sidenafil citrate) **EXPIRED** | Pfizer limited | Treatment of pulmonary arterial hypertension. Revatio has been shown to improve exercise ability and to reduce mean pulmonary arterial pressure | |
| **2006** | | | | | |
| 31 | 23 | **Naglazyme** (N-acetylgalactosamine 4-sulfatase,A) **EXPIRED** | BioMarin Europe | Long term enzyme replacement therapy in patients with a confirmed diagnosis of Mucopolysaccharidosis VI (MPS VI; (N-acetylgalactosamine 4-sulfatase deficiency; Maroteaux Lamy syndrome) | |
| 32 | 24 | **Myozyme** (recombinant human acid alpha-glucosidase **EXPIRED** | Genzyme Europe | Myozyme is indicated for long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of Pompe disease (acid alpha-glucosidase deficiency).<br>Myozyme is indicated in adults and paediatric patients of **all ages.**<br>In patients with late-onset Pompe disease the evidence of efficacy is limited | |
| 33 | 25 | **Evoltra** (clofarabine) **EXPIRED** | Genzyme Europe BV | Treatment of acute lymphoblastic leukaemia (ALL) in **paediatric** patients who have relapsed or are refractory after receiving at least two prior regimens and where there is no other treatment option anticipated to result in a durable response. Safety and efficacy have been assessed in studies of patients ≤ 21 years old at initial diagnosis | |
| 34 | 26 | **Nexavar** (sorafenib tosylate) **EXPIRED** | Bayer Healthcare AG | For the treatment of patients with advanced renal cell carcinoma who have failed prior interferon-alpha or interleukin-2 based therapy or are considered unsuitable for such therapy | |
| 35 | | **Nexavar** (sorafenib tosylate) **EXPIRED** | Bayer Healthcare AG | Extension of Indication to include treatment of hepatocellular carcinoma. | 2007 |
| 36 | | **Nexavar** (sorafenib tosylate) | Bayer Healthcare AG | Extension of indication for the treatment of patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma, refractory to radioactive iodine. 5/2024 | 2014 |
| 37 | 27 | **Savene** (dexrazoxane) **EXPIRED** | SpePharm Holding BV | Treatment of anthracycline extravasation | |
| 38 | 28 | **Exjade** (4-(3,5-Bis (hydroxiphenyl) -1,2,4) triazol-1-yl)benzoic acid, B) **EXPIRED** | Novartis Europharm Limited | Treatment of chronic iron overload due to blood transfusions (transfusion haemosiderosis) in adult and **paediatric** patients (aged 2 years and over) | |

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| 39 | 29 | **Sprycel** (dasatinib) **EXPIRED** | Bristol-Myers Squibb Pharma | Treatment of adults with Philadelphia chromosome positive (Ph+) acute lymphoblastic leukaemia (ALL) and lymphoid blast CML with resistance or intolerance to prior therapy.<br>Treatment of adults with chronic, accelerated or blast phase chronic myeloid leukaemia (CML) with resistance or intolerance to prior therapy including imatinib mesilate. | |
| 40 | 30 | **Sutent** (sunitinib) **WITHDRAWN** | Pfizer Ltd. | Sutent is indicated for the treatment of advanced and/or metastatic renal cell carcinoma.<br>Sutent is indicated for the treatment of unresectable and/or metastatic malignant gastrointestinal stromal tumour (GIST) after failure of imatinib mesylate treatment due to resistance or intolerance. | |
| 41 | 31 | **Thelin** (sitaxentan sodium) **WITHDRAWN FROM THE MARKET - SAFETY** | Pfizer Limited. | Treatment of patients with pulmonary arterial hypertension classified as WHO functional class III, to improve exercise capacity. Efficacy has been shown in primarily pulmonary hypertension and in pulmonary hypertension associated with connective tissue disease. | |
| **2007** | | | | | |
| 42 | 32 | **Diacomit** (stiripentol) **EXPIRED** | BIOCODEX | Indicated for use in conjunction with clobazam and valproate as adjunctive therapy of refractory generalized tonic-clonic seizures in patients with severe myoclonic epilepsy in **infancy** (SMEI, Dravet's syndrome) whose seizures are not adequately controlled with clobazam and valproate. | |
| 43 | 33 | **Elaprase** (iduronate-2-sulfatase) **EXPIRED** | Shire Human Genetic Therapies AB - Sweden | "Elaprase is indicated for the long-term treatment of patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II).<br>Heterozygous females were not studied in the clinical trials." | |
| 44 | 34 | **Inovelon** (rufinamide) **EXPIRED** | Esai Limited | Adjunctive therapy in the treatment of seizures associated with Lennox-Gastaut syndrome in patients **4 years** and older. | |
| 45 | 35 | **Cystadane** (betaine anhydrous A) **EXPIRED** | Orphan Europe | Adjunctive treatment of homocystinuria, involving deficiencies or defects in:<br>- cystathionine beta-synthase (CBS),<br>- 5,10-methylene-tetrahydrofolate reductase (MTHFR),<br>- cobalamin cofactor metabolism (cbl).<br>Cystadane should be used as supplement to other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a specific diet. | |
| 46 | 36 | **Revlimid** (lenalidomide) **EXPIRED** | Celgene Europe Ltd | Revlimid is indicated for the treatment of adult patients with previously untreated multiple myeloma who are not eligible for transplant.<br>Revlimid in combination with dexamethasone is indicated for the treatment of multiple myeloma in adult patients who have received at least one prior therapy. | |

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| 47 | | **Revlimid** (lenalidomide) | Celgene Europe Limited | Revlimid is indicated for the treatment of patients with transfusion-dependent anaemia due to low- or intermediate-1-risk myelodysplastic syndromes associated with an isolated deletion 5q cytogenetic abnormality when other therapeutic options are insufficient or inadequate. 06/2023 | 2013 |
| 48 | | **Revlimid** (lenalidomide) | Celgene Europe Limited | Revlimid as monotherapy is indicated for the treatment of adult patients with relapsed or refractory mantle cell lymphoma 07/2026 | 2016 |
| 49 | 37 | **Soliris** (eculizumab) **EXPIRED** | Alexion Europe | Indicated in adults and **children** for the treatment of patients with paroxysmal nocturnal haemoglobinuria (PNH). Evidence of clinical benefit demonstrated in patients with haemolysis with clinical symptom(s) indicative of high disease activity, regardless of transfusion history | |
| 50 | | **Soliris** (eculizumab) | Alexion Europe SAS - France | Extension Of Indication for atypical haemolytic uremic syndrome (aHUS) 11/2023 | 2011 |
| 51 | | **Soliris** (eculizumab) | Alexion Europe SAS - France | Extension Of Indication for Refractory generalized myasthenia gravis (gMG) in patients who are anti-acetylcholine receptor (AChR) antibody-positive 08/2027 | 2017 |
| 52 | 38 | **Siklos** (hydroxycarbamide) | Addmedica SAS - France | "Indicated for the prevention of recurrent painful vaso-occlusive crises including acute chest syndrome in adults, adolescents and **children** older than 2 years suffering from symptomatic Sickle Cell Syndrome." | |
| 53 | 39 | **Increlex** (mecasermin) **EXPIRED** | Ipsen Pharma | Long-term treatment of growth failure in **children** and adolescents with severe primary insulin like growth factor-1 deficiency (Primary IGFD).<br>Severe Primary IGFD is defined by:<br>• height standard deviation score ≤ –3.0 and<br>• basal IGF-1 levels below the 2.5th percentile for age and gender and<br>• GH sufficiency.<br>• Exclusion of secondary forms of IGF-1 deficiency, such as malnutrition, hypothyroidism, or chronic treatment with pharmacologic doses of anti-inflammatory steroids.<br>Severe Primary IGFD includes patients with mutations in the GH receptor (GHR), post-GHR signalling pathway, and IGF-1 gene defects; they are not GH deficient, and therefore, they cannot be expected to respond adequately to exogenous GH treatment. It is recommended to confirm the diagnosis by conducting an IGF-1 generation test. | |
| 54 | 40 | **Atriance** (nelarabine) **EXPIRED** | Glaxo Group Ltd | Treatment of patients with T-cell acute lymphoblastic leukaemia (T-ALL) and T-cell lymphoblastic lymphoma (T-LBL) whose disease has not responded to or has relapsed following treatment with at least two chemotherapy regimens.<br>Due to the small patient populations in these disease settings, the information to support these indications is based on limited data. | |
| 55 | 41 | **Gliolan** (5 aminolevulinic | Medac GmbH | Visualisation of malignant tissue during surgery for malignant glioma | |

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| | | acid hydrochloride L) **EXPIRED** | | | |
| 56 | 42 | **Yondelis** (trabectedin) **EXPIRED** | PharmaMar SA | Treatment of patients with advanced soft tissue sarcoma, after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on liposarcoma and leiomyosarcoma patients | |
| 57 | | **Yondelis** (trabectedin) | PharmaMar SA | EXTENSION OF INDICATION<br>Indicated for the treatment of patients with advanced soft tissue sarcoma, after failure of anthracyclines and ifosfamide, or who are unsuited to receive these agents. Efficacy data are based mainly on liposarcoma and leiomyosarcoma patients.<br>In combination with pegylated liposomal doxorubicin (PLD) is indicated for the treatment of patients with relapsed platinum-sensitive ovarian cancer.<br>30/10/2019 | 2009 |
| 58 | 43 | **Torisel** (temsirolimus) **EXPIRED** | Pfizer Limited | First-line treatment of patients with advanced renal cell carcinoma who have at least three of six prognostic risk factors. | |
| 59 | | **Torisel** (temsiroliums) | Pfizer Limited | EXTENSION OF INDICATION to include treatment of adult patients with relapsed and/or refractory mantle cell lymphoma.<br>25/08/2019 | 2009 |
| 60 | 44 | **Tasigna** (nilotinib) | Novartis Europharm Ltd | Treatment of Philadelphia chromosome positive chronic myelogenous leukaemia (CML) – *2 additional years of market exclusivity as paediatric reward granted on 17 Nov 2017 – will expire in 21/11/2019* | |
| **2008** | | | | | |
| 61 | 45 | **Thalidomide Celgene** (thalidomide) **EXPIRED** | Celgene Europe Limited | Thalidomide Celgene in combination with melphalan and prednisone as first line treatment of patients with untreated multiple myeloma, aged ≥ 65 years or ineligible for high dose chemotherapy. | |
| 62 | 46 | **Volibris** (ambrisentan) **EXPIRED** | Glaxo Group Ltd | Treatment of patients with pulmonary arterial hypertension (PAH) classified as WHO functional class II and III, to improve exercise capacity. | |
| 63 | 47 | **Firazyr** (icatibant acetate L) | Shire Orphan Therapies GmbH | Indicated for symptomatic treatment of acute attacks of hereditary angioedema (HAE) in adults (with C1-esterase-inhibitor deficiency). - *2 additional years of market exclusivity as paediatric reward granted on 15/07/2020* | |
| 64 | 48 | **Ceplene** (histamine dihydrochloride ) **EXPIRED** | Meda AB | Ceplene maintenance therapy is indicated for adult patients with acute myeloid leukaemia in first remission concomitantly treated with interleukin-2 (IL-2). The efficacy of Ceplene has not been fully demonstrated in patients older than age 60. | |
| 65 | 49 | **Kuvan** (sapropterin dihydrochloride ) | Merck Serono Europe Limited | Treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients of 4 years of age and over with phenylketonuria (PKU) who have been shown to be responsive to such treatment. Kuvan is also indicated for the treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients with | |

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| | | | | tetrahydrobiopterin (BH4) deficiency who have been shown to be responsive to such treatment. *2 additional years of market exclusivity as paediatric reward granted on 24/06/2015-expires 4/12/2020* | |
| 66 | 50 | **Vidaza** (azacitidine) **EXPIRED** | Celgene Europe Ltd-United Kingdom | Treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with:<br>• intermediate-2 and high-risk myelodysplastic syndromes (MDS) according to the International Prognostic Scoring System (IPSS),<br>• chronic myelomonocytic leukaemia (CMML) with 10-29% marrow blasts without myeloproliferative disorder,<br>• acute myeloid leukaemia (AML) with 20-30% blasts and multi-lineage dysplasia, according to World Health Organisation (WHO) classification. | |
| **2009** | | | | | |
| 67 | 51 | **Nplate** (romiplostim) **EXPIRED** | Amgen Europe BV | Indicated for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients refractory to other treatments (e.g. corticosteroids, immunoglobulins). Nplate may be considered as second line treatment for adult non-splenectomised patients where surgery is contra-indicated. | |
| 68 | 52 | **Mepact** (mifamurtide) **EXPIRED** | Takeda France SAS | Indicated in **children**, adolescents and young adults for the treatment of high-grade resectable non-metastatic osteosarcoma after macroscopically complete surgical resection. It is used in combination with post-operative multi-agent chemotherapy | |
| 69 | 53 | **Peyona** (previously Nymusa, caffeine citrate | Chiesi Farmaceutici S.P.A. - Italy | Treatment of primary apnoea of premature **F** | |
| 70 | 54 | **Mozobil** (plerixafor) | Genzyme BV The Netherlands | Indicated in combination with G-CSF to enhance mobilisation of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in adults patients with lymphoma and multiple myeloma whose cells mobilise poorly. 4/08/2019 | |
| 71 | 55 | **Cayston** (aztreonam lysinate inhalation use) | Gilead Sciences International Ltd – UK | Suppressive therapy of chronic pulmonary infections due to *Pseudomonas aeruginosa* in patients with cystic fibrosis (CF) aged 6 years and older. 23/09/2019 | |
| 72 | 56 | **Rilonacept Regeneron** (formerly Arcalyst; rilonacept) **WITHDRAWN FROM THE MARKET – SAFETY** | Regeneron UK | Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) including Familial Cold Autoinflammatory Syndrome (FCAS) and Muckle-Wells Syndrome (MWS) in adults and **children** aged 12 years and older. | |
| 73 | 57 | **Firdapse** (amifampridine) | BioMarin Europe Ltd | Symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults. 28/12/2019 | |

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| 74 | 58 | **Revolade** (eltrombopag) **WITHDRAWN** | GlaxoSmithKline Trading Services Limited – Ireland | Indicated for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins). Revolade may be considered as second line treatment for adult non-splenectomised patients where surgery is contraindicated. | |
| 75 | 59 | **Afinitor** (everolimus) **WITHDRAWN** | Novartis Europharm Ltd | Treatment of patients with advanced renal cell carcinoma, whose disease has progressed on or after treatment with VEGF-targeted therapy. | |
| 76 | 60 | **Ilaris** (canakinumab) **WITHDRAWN** | Novartis Europharm Ltd. | Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) in adults, adolescents and **children** aged 4 years and older with body weight above 15 kg, including Muckle-Wells Syndrome (MWS), Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA), Severe forms of Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash. | |
| **2010** | | | | | |
| 77 | 61 | **Tepadina** (thiotepa) | Adienne S.r.l - Italy | Indicated, in combination with other chemotherapy medicinal products:<br>1) with or without total body irradiation (TBI), as conditioning treatment prior to allogeneic or autologous haematopoietic progenitor cell transplantation (HPCT) in haematological diseases in adult and **paediatric patients;**<br>2) when high dose chemotherapy with HPCT support is appropriate for the treatment of solid tumours in adult and paediatric patients." 03/2020 | |
| 78 | 62 | **Arzerra** (ofatumumab) **WITHDRAWN** | Glaxo Group Limited - UK | Refractory chronic lymphocytic leukaemia (CLL):<br>Arzerra is indicated for the treatment of CLL in patients who are refractory to fludarabine and alemtuzumab | |
| 79 | | **Arzerra** (ofatumumab) **WITHDRAWN** | Glaxo Group Limited - UK | Previously untreated chronic lymphocytic leukaemia (CLL):<br>Arzerra in combination with chlorambucil or bendamustine is indicated for the treatment of patients with CLL who have not received prior therapy and who are not eligible for fludarabine-based therapy. | 2014 |
| 80 | 63 | **VPRIV** (velaglucerase alfa) | Shire Pharmaceuticals Ireland Limited – Ireland | Treatment of type 1 Gaucher disease. *2 additional years of market exclusivity as paediatric reward granted on 29/08/2016*<br>08/2022 | |
| **2011** | | | | | |
| 81 | 64 | **Esbriet** (perfenidone) | InterMune UK Ltd. | Treatment for adults with idiopathic pulmonary fibrosis 03/2021 | |
| 82 | 65 | **TOBI podhaler** (tobramycin) | Novartis Europharm | Suppressive therapy of chronic pulmonary infection due to *Pseudomonas aeruginosa* in adults and **children** aged 6 years and older | |

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| | | | Limited | with cystic fibrosis 07/2023 | |
| 83 | 66 | **Votubia** (everolimus) | Novartis Europharm Limited | Treatment of patients aged **3 years** and older with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex (TSC) who require therapeutic intervention but are not amenable to surgery 09/2021 | |
| 84 | 67 | **Plenadren** (hydrocortisone (modified release tablet) | ViroPharma SPRL | Treatment for adults with adrenal insufficiency 11/2021 | |
| 85 | 68 | **Vyndaqel** (tafamidis) | Pfizer Limited - UK | Treatment of transthyretin amyloidosis in adult patients with symptomatic polyneuropathy 11/2021 | |
| **2012** | | | | | |
| 86 | 69 | **Xaluprine** (previously known as Mercaptopurine Nova Laboratories and Novapurine) | Nova Laboratories Limited - UK | Treatment of acute lymphoblastic leukaemia (ALL) in adults, adolescents and **children** 03/2022 | |
| 87 | 70 | **Bronchitol** (manitolum) | Pharmaxis Pharmaceuticals | Treatment of cystic fibrosis in adults aged 18 years and above as an add-on therapy to best standard of care 04/2022 | |
| 88 | 71 | **Signifor** (pasireotide) | Novartis Europharm Limited UK | Treatment of adult patients with Cushing's disease for whom surgery is not an option or for whom surgery has failed 04/2022 | |
| 89 | | **Signifor** (pasireotide) | Novartis Europharm Limited UK | Treatment of adult patients with acromegaly for whom surgery is not an option or has not been curative and who are inadequately controlled on treatment with another somatostatin analogue 11/2024 | 2014 |
| 90 | 72 | **Kalydeco** ivacaftor | Vertex Pharmaceuticals (U.K.) Limited | Treatment of cystic fibrosis (CF) in patients aged **12 months** (granules) and older who have mutation in the CFTR gene 07/2022 | |
| 91 | 73 | **Jakavi** (ruxolitinib) **WITHDRAWN** | Novartis Europharm Limited - UK | Treatment of disease related splenomegaly or symptoms in adult patients with primary myelofibrosis (also known as chronic idiopathic myelofibrosis), post polycythaemia vera myelofibrosis or post essential thrombocythaemia myelofibrosis. | |
| 92 | 74 | **Revestive** (teduglutide) | NPS Pharma Holdings Limited | Treatment of patients **aged 1 year and above** with Short Bowel Syndrome. Patients should be stable following a period of intestinal adaptation after surgery 09/2022 | |
| 93 | 75 | **NovoThirteen** (catridecacog) **WITHDRAWN** | Novo Nordisk A/S | Long-term prophylactic treatment of bleeding in adult and paediatric patients 6 years and above with congenital factor-XIII-A-subunit deficiency. | |
| 94 | 76 | **Dacogen** (decitabine) | Janssen-Cilag International NV - Belgium | Treatment of adult patients aged 65 years and above with newly diagnosed de novo or secondary acute myeloid leukaemia (AML), according to the WHO classification, who are not candidates for standard induction | |

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| | | | | chemotherapy" 09/2022 | |
| 95 | 77 | **Glybera** (adeno-associated viral vector expressing lipoprotein lipase | uniQure biopharma B.V. - The Netherlands | Glybera is indicated for adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein 09/2022 | |
| 96 | 78 | **Adcetris** (brentuximab vedotin) | Takeda Pharma A/S, Danmark | Adcetris is indicated for the treatment of adult patients with relapsed or refractory CD30+ H83 (Hodgkin's lymphoma):<br>1.following autologous stem-cell transplant (ASCT) or;<br>2.following at least two prior therapies when ASCT or multi-agent chemotherapy is not a treatment option 10/2022 | |
| 97 | | **Adcetris** (brentuximab vedotin) | Takeda Pharma A/S, Danmark | Adcetris is indicated for the treatment of adult patients with relapsed or refractory systemic anaplastic large cell lymphoma (sALCL) 10/2022 | |
| 98 | | **Adcetris** (brentuximab vedotin) | Takeda Pharma A/S, Danmark | ADCETRIS is indicated for the treatment of adult patients with CD30+ cutaneous T-cell lymphoma (CTCL) after at least 1 prior systemic therapy 12/2027 | 2017 |
| 99 | 79 | **NexoBrid** (concentrate of proteolytic enzymes enriched in bromelain) | MediWound Germany GmbH | NexoBrid is indicated for removal of eschar in adults with deep partial- and full-thickness thermal burns 12/2022 | |
| **2013** | | | | | |
| 100 | 80 | **Bosulif** (bosutinib)<br>**WITHDRAWN** | Pfizer Limited United Kingdom | Treatment of chronic myeloid leukaemia | |
| 101 | 81 | **Iclusig** (ponatinib) | ARIAD Pharma Ltd - UK | Iclusig is indicated in adult patients with:<br>1) chronic-phase, accelerated-phase or blast-phase chronic myeloid leukaemia (CML) who are resistant to dasatinib or nilotinib, who are intolerant to dasatinib or nilotinib and for whom subsequent treatment with imatinib is not clinically appropriate, or who have the T315I mutation; 07/2023 | |
| 102 | | **Iclusig** (ponatinib) | ARIAD Pharma Ltd - UK | 2) Philadelphia-chromosome-positive acute lymphoblastic leukaemia (Ph+ ALL) who are resistant to dasatinib, who are intolerant to dasatinib and for whom subsequent treatment with imatinib is not clinically appropriate, or who have the T315I mutation 07/2023 | |
| 103 | 82 | **Imnovid** (previously Pomalidomide Celgene; pomalidomide) | Celgene Europe Limited - UK | Pomalidomide Celgene in combination with dexamethasone is indicated in the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least two prior treatment regimens, including both lenalidomide and bortezomib, and have demonstrated disease progression on the last therapy 08/2023 | |
| 104 | 83 | **Procysbi** (cysteamine bitartrate) | Raptor Pharmaceuticals Europe BV - The | Probysbi is indicated for the treatment of proven nephropathic cystinosis. Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| | | | Netherlands | development of renal failure 08/2023 | |
| 105 | 84 | **Orphacol** (cholic acid) | Laboratoires CTRS | Orphacol is indicated for the treatment of inborn errors in primary bile-acid synthesis due to 3β-hydroxy-Δ5-C27-steroid oxidoreductase deficiency or Δ4-3-oxosteroid-5β-reductase deficiency in infants, **children** and adolescents aged 1 month to 18 years and adults 09/2023 | |
| 106 | 85 | **Defitelio** (defibrotide) | Gentium S.p.A. - Italy | Defitelio is indicated for the treatment of severe hepatic veno-occlusive disease (VOD) also known as sinusoidal obstructive syndrome (SOS) in haematopoietic stem-cell transplantation (HSCT) therapy. It is indicated in adults and in adolescents, **children** and infants over 1 month of age 10/2023 | |
| 107 | 86 | **Opsumit** (macitentan) | Janssen-Cilag International NV | Opsumit, as monotherapy or in combination, is indicated for the long-term treatment of pulmonary arterial hypertension (PAH) in adult patients of WHO Functional Class (FC) II to III 12/2023 | |
| 108 | 87 | **Jinarc** (tolvaptan) **WITHDRAWN** | Otsuka Pharmaceutical Europe Ltd | Autosomal dominant polycystic kidney disease | |
| **2014** | | | | | |
| 109 | 88 | **Sirturo** (bedaquiline fumarate) | Janssen-Cilag International N.V. Belgium | Indicated for use as part of an appropriate combination regimen for pulmonary multidrug resistant tuberculosis (MDR TB) in adult patients when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability 03/2024 | |
| 110 | 89 | **Cometriq** (cabozantinib) | TMC Pharma | Treatment of adult patients with progressive, unresectable locally advanced or metastatic medullary thyroid carcinoma 03/2024 | |
| 111 | 90 | **Adempas** (riociguat) | Bayer Pharma AG | Treatment of adult patients with Chronic thromboembolic pulmonary hypertension (CTEPH) and Pulmonary arterial hypertension (PAH) 03/2024 | |
| 112 | 91 | **Kolbam** (cholic acid) **WITHDRAWN** | FGK Representative Service GmbH, Germany | Inborn errors in primary bile-acid synthesis | |
| 113 | | **Kolbam** (cholic acid) | Retrophin Europe Ltd | Inborn errors in primary bile-acid synthesis due to Sterol 27-hydroxylase (presenting as cerebrotendinous xanthomatosis, CTX) deficiency, 2- (or α-) methylacyl-CoA racemase (AMACR) deficiency or Cholesterol 7α-hydroxylase (CYP7A1) deficiency in infants, **children** and adolescents aged 1 month to 18 years and adults 11/2025 | 2015 |
| 114 | 92 | **Granupas** (previously para-aminosalicylic acid Lucane or PAS-GR) | Lucane Pharma SA - France | Treatment of tuberculosis in adults and **paediatric** patients from 28 days of age and older when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability 04/2024 | |
| 115 | 93 | **Deltyba** (delamanid) | Otsuka Novel Products GmbH - | Treatment of multidrug-resistant tuberculosis (MDR-TB) in adults 04/2024 | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| | | | Germany | | |
| 116 | 94 | **Vimizim** (elosulfase alfa) | BioMarin Europe Ltd | Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA) in patients of **all ages** 04/2024 | |
| 117 | 95 | **Sylvant** (siltuximab) | Janssen-Cilag International NV | Treatment of adult patients with multicentric Castleman's disease (MCD who are human immunodeficiency virus (HIV) negative and human herpesvirus-8 (HHV-8) negative 05/2024 | |
| 118 | 96 | **Gazyvaro** (obinutuzumab) | Roche Registration Ltd | Gazyvaro in combination with chlorambucil is indicated for the treatment of adult patients with previously untreated chronic lymphocytic leukaemia (CLL) and with comorbidities making them unsuitable for full-dose fludarabine based therapy 07/2024 | |
| 119 | | **Gazyvaro** (obinutuzumab) | Roche Registration Ltd | Gazyvaro in combination with chemotherapy, followed by Gazyvaro maintenance therapy in patients achieving a response, is indicated for the treatment of patients with previously untreated advanced Follicular lymphoma FL 06/2026 | 2016 |
| 120 | 97 | **Translarna** (ataluren) | PTC Therapeutics Limited | Translarna is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged **5 years** and older. Efficacy has not been demonstrated in non-ambulatory patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing 08/2024 | |
| 121 | 98 | **Imbruvica** (ibrutinib) | Janssen-Cilag International NV | Imbruvica is indicated for the treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL) 10/2024 | |
| 122 | | **Imbruvica** (ibrutinib) | Janssen-Cilag International NV | Imbruvica is indicated for the treatment of adult patients with chronic lymphocytic leukaemia (CLL) who have received at least one prior therapy, or in first line in the presence of 17p deletion or TP53 mutation in patients unsuitable for chemo immunotherapy 10/2024 | |
| 123 | | **Imbruvica** (ibrutinib) | Janssen-Cilag International NV | Imbruvica is indicated for the treatment of adult patients with Waldenström's macroglobulinaemia (WM) who have received at least one prior therapy, or in first line treatment for patients unsuitable for chemo-immunotherapy 07/2025 | 2015 |
| 124 | 99 | **Ketoconazole HRA** (ketoconazole) | Laboratoire HRA Pharma | Ketoconazole HRA is indicated for the treatment of endogenous Cushing's syndrome in adults and adolescents above the **age of 12 years** 11/2024 | |
| 125 | 100 | **Lynparza** (olaparib) **WITHDRAWN** | AstraZeneca AB | Lynparza is indicated as monotherapy for the maintenance treatment of adult patients with platinum-sensitive relapsed BRCA-mutated (germline and/or somatic) high grade serous epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete response or partial response) to platinum-based chemotherapy | |
| 126 | 101 | **Cyramza** (ramucirumab) **WITHDRAWN** | Eli Lilly Nederland B.V. | In combination with paclitaxel is indicated for the treatment of adult patients with advanced gastric cancer or gastro-oesophageal junction adenocarcinoma with disease progression after prior platinum and | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| | | | | fluoropyrimidine chemotherapy.<br>Monotherapy is indicated for the treatment of adult patients with advanced gastric cancer or gastro-oesophageal junction adenocarcinoma with disease progression after prior platinum or fluoropyrimidine chemotherapy, for whom treatment in combination with paclitaxel is not appropriate. | |
| 127 | 102 | **Scenesse** (afamelanotide) | Clinuvel UK Limited | Prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP) 12/2024 | |
| **2015** | | | | | |
| 128 | 103 | **Ofev** (nibtedanib) | Boehringer Ingelheim Pharma GmbH & Co. KG | Ofev is indicated in adults for the treatment of Idiopathic Pulmonary Fibrosis (IPF) 01/2025 | |
| 129 | 104 | **Cerdelga** (eliglustat) | Genzyme Europe BV | Cerdelga is indicated for the long-term treatment of adult patients with Gaucher disease type 1 (GD1), who are CYP2D6 poor metabolisers (PMs), intermediate metabolisers (IMs) or extensive metabolisers (EMs) 01/2025 | |
| 130 | 105 | **Holoclar** (*ex vivo* expanded autologous human corneal epithelial cells containing stem cells) | Chiesi Farmaceutici S.p.A. | Treatment of adult patients with moderate to severe limbal stem cell deficiency (defined by the presence of superficial corneal neovascularisation in at least two corneal quadrants, with central corneal involvement, and severely impaired visual acuity), unilateral or bilateral, due to physical or chemical ocular burns. A minimum of 1-2 mm2 of undamaged limbus is required for biopsy 02/2025 | |
| 131 | 106 | **Lenvima** (lenvatinib mesylate) **WITHDRAWN** | Eisai Europe Ltd | Lenvima is indicated for the treatment of adult patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma (DTC), refractory to radioactive iodine (RAI) | |
| 132 | | **Lenvima** (lenvatinib mesylate) **WITHDRAWN** | Eisai Europe Ltd | Lenvima is indicated as monotherapy for the treatment of adult patients with advanced or unresectable hepatocellular carcinoma (HCC) | |
| 133 | 107 | **Hetlioz** (tasimelteon) | Vanda Pharmaceuticals Ltd | Hetlioz is indicated for the treatment of Non-24-Hour Sleep-Wake Disorder (Non-24) in totally blind adults 07/2025 | |
| 134 | 108 | **Unituxin** (dinutuximab) **WITHDRAWN** | United Therapeutics Europe Ltd | Unituxin is indicated for the treatment of high-risk neuroblastoma in **children** aged 12 months to 17 years. who have previously received induction chemotherapy and achieved at least a partial response, followed by myeloablative therapy and autologous stem cell transplantation. It is administered in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), interleukin-2 (IL-2), and isotretinoin | |
| 135 | 109 | **Kanuma** (sebelipase alfa) | Synageva BioPharma Ltd | Kanuma is used to treat patients of **all ages** with lysosomal acid lipase deficiency 9/2025 | |
| 136 | 110 | **Farydak** (panobinostat ) | Novartis Europharm Ltd | Farydak is indicated in combination with bortezomib and dexamethasone, for the treatment of relapsed and/or refractory multiple myeloma in adults patients who | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| | | | | have received at least two prior regimens including bortezomib and an immunomodulatory agent 9/2025 | |
| 137 | 111 | **Strensiq** (asfotase alfa) | Alexion Europe SAS | Strensiq is indicated for long-term enzyme replacement therapy in patients with **paediatric**-onset hypophosphatasia to treat the bone manifestations of the disease 9/2025 | |
| 138 | 112 | **Raxone** (ibedenone) | Santera Pharmaceuticals Gmbh | Raxone is indicated for the treatment of visual impairment in **adolescent** and adult patients with Leber's Hereditary Optic Neuropathy 10/2025 | |
| 139 | 113 | **Cresemba** (isavuconazole) | Basilea Medical Ltd | Cresemba is indicated for the treatment of adults with invasive aspergillosis 10/2025 | |
| 140 | | **Cresemba** (isavuconazole) | Basilea Medical Ltd | Treatment of mucormycosis in patients for whom amphotericin B is inappropriate 10/2025 | |
| 141 | 114 | **Kyprolis** (carfilzomib) | Amgen Europe BV | Kyprolis is indicated for the treatment of adult patients with multiple myeloma 11/2025 | |
| 142 | 115 | **Orkambi** (lumacaftor/ ivacaftor) **WITHDRAWN** | Vertex Pharmaceuticals | Orkambi is indicated for the treatment of cystic fibrosis | |
| 143 | 116 | **Obizur** (susoctog alfa) **WITHDRAWN** | Baxalta Innovations GmbH | Obizur is indicated for the treatment of haemophilia A | |
| 144 | 117 | **Elocta** (efmoroctocog alfa) **WITHDRAWN** | Biogen Idec Ltd | Elocta is indicated for the treatment of haemophilia A | |
| 145 | 118 | **Blincyto** (blinatumomab) | Amgen Europe B.V. | Blincyto is indicated as monotherapy for the treatment of **paediatric patients** aged 1 year or older with Philadelphia chromosome negative CD19 positive B-precursor ALL which is refractory or in relapse after receiving at least two prior therapies or in relapse after receiving prior allogeneic hematopoietic stem cell transplantation 11/2025 | |
| 146 | 119 | **Ravicti** (glycerol phenylbutyrate) | Horizon Therapeutics Limited | Ravicti is indicated for the treatment of inborn urea cycle disorders (UCDs), including CPS, OTC, ASS, ASL, ARG and HHH 11/2025 | |
| 147 | 120 | **Quinsair** (levofloxacin) **WITHDRAWN** | Regintel | Cystic fibrosis | |
| **2016** | | | | | |
| 148 | 121 | **Coagadex** (human coagulation factor X) | Bio Products Laboratory limited | Treatment and prophylaxis of bleeding episodes and for perioperative management in **all age groups** patients with hereditary factor X deficiency 03/2026 | |
| 149 | 122 | **Wakix** (pitolisant) | Bioprojet Pharma | Wakix is indicated in adults for the treatment of narcolepsy with or without cataplexy 04/2026 | |
| 150 | 123 | **Idelvion** (albutrepenonacog alfa) | CSL Behring GmbH | Treatment and prophylaxis of bleeding in **all age groups** patients with haemophilia B (congenital factor IX | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| | | | | deficiency) 06/2026 | |
| 151 | 124 | **Uptravi** (selexipag) **WITHDRAWN** | Actelion Registration Ltd | Pulmonary arterial hypertension | |
| 152 | 125 | **Alprolix** (eftrenacog alfa) | Biogen Idec Ltd | Treatment and prophylaxis of bleeding in **all age groups** patients with haemophilia B (congenital factor IX deficiency) 05/2026 | |
| 153 | 126 | **Darzalex** (daratumumab) | Janssen-Cilag International N.V. | Treatment of plasma cell myeloma in adults patients 05/2026 | |
| 154 | 127 | **Galafold** (migalastat hydrochloride) | Amicus Therapeutics UK Ltd | Galafold is indicated for long-term treatment of adults and **adolescents** aged 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation 05/2026 | |
| 155 | 128 | **Strimvelis** (autologous CD34+ + cells transduced with retroviral vector encoding for the human adenosine deaminase (ADA)) | GlaxoSmithKline Trading Services Limited | Severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID) 05/2028 | |
| 156 | 129 | **Zalmoxis** (allogeneic T cells genetically modified with a retroviral vector encoding for a truncated form of the human low affinity nerve growth factor receptor and the herpes simplex I virus thymidine kinase) | MolMed SpA | Adjunctive treatment in haematopoietic stem cell transplantation (HSCT) of adult patients with high-risk haematological malignancies 08/2026 | |
| 157 | 130 | **Onivivyde** (irinotecan hydrochloride trihydrate) | Baxalta Innovations GmbH | Metastatic adenocarcinoma of the pancreas, in combination with 5 fluorouracil (5 FU) and leucovorin (LV), in adult patients who have progressed following gemcitabine based therapy 10/2026 | |
| 158 | 131 | **Lartruvo** (olaratumab) **WITHDRAWN FROM THE MARKET** | Eli Lilly Nederland B.V. | Treatment of adult patients advanced soft tissue sarcoma 11/2026 | |
| 159 | 132 | **Ninlaro** (ixazomib) | Takeda Pharma A/S | NINLARO in combination with lenalidomide and dexamethasone is indicated for the treatment of adult patients with multiple myeloma who have received at least one prior therapy 11/2026 | |
| 160 | 133 | **Venclyxto** (venetoclax) **WITHDRAWN** | AbbVie Ltd | Chronic lymphocytic leukemia | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| 161 | 134 | **Ocaliva** (obeticholic acid) | Intercept Pharma Ltd | Primary biliary cholangitis in adults 12/2026 | |
| 162 | 135 | **SomaKit TOC** (edotreotide) | Advanced Accelerator Applications | Diagnosis of gastro-entero-pancreatic neuroendocrine tumours 12/2026 | |
| **2017** | | | | | |
| 163 | 136 | **Cystadrops** (mercaptamine) | Orphan Europe S.A.R.L. | Treatment of corneal cystine crystal deposits in adults and **children** from 2 years of age with cystinosis 01/2027 | |
| 164 | 137 | **Ledaga** (chlormethine) | Actelion Registration Ltd | Ledaga is indicated for the topical treatment of mycosis fungoides-type cutaneous T-cell lymphoma (MF type CTCL) in adult patients 03/2027 | |
| 165 | 138 | **Natpar** (parathyroid hormone) | Shire Pharmaceutic als Ireland Ltd | Natpar is indicated as adjunctive treatment of adult patients with chronic hypoparathyroidism who cannot be adequately controlled with standard therapy alone 04/2027 | |
| 166 | 139 | **Qarziba** *(previously Dinutuximab beta EUSA and Dinutuximab beta Apeiron)* (dinutuximab beta) | EUSA Pharma (Netherlands) B.V. | Neuroblastoma (in patients over **1 year** of age) 11/2027 | |
| 167 | 140 | **Spinraza** (nusinersen) | Biogen Idec Ltd | 5q Spinal muscular atrophy 06/2027 | |
| 168 | 141 | **Brineura** (cerliponase alfa) | Biomarin International Limited | Neuronal ceroid lipofuscinosis type 2 (CLN2) disease 06/2027 | |
| 169 | 142 | **Besponsa** (inotuzumab ozogamicin) | Pfizer Limited | Treatment of adults with Philadelphia chromosome positive (Ph+) relapsed or refractory CD22-positive B cell precursor acute lymphoblastic leukaemia (ALL) 03/2027 | |
| 170 | 143 | **Oxervate** (recombinant human nerve growth factor - cenegermin) | Dompe farmaceutici s.p.a. | Treatment of moderate (persistent epithelial defect) or severe (corneal ulcer) neurotrophic keratitis in adults 10/2027 | |
| 171 | 144 | **Bavencio** (avelumab) | Merck Serono Europe Limited | Bavencio is indicated as monotherapy for the treatment of adult patients with metastatic Merkel cell carcinoma (MCC) 09/2027 | |
| 172 | 145 | **Rydapt®** (midostaurin) | Novartis Europharm Ltd | Adult patients with acute myeloid leukemia (AML) 09/2027 | |
| 173 | | **Rydapt®** (midostaurin) | Novartis Europharm Ltd | Adult patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with associated haematological neoplasm (SM AHN), or mast cell leukaemia (MCL) 09/2027 | |
| 174 | 146 | **Xermelo®** (telotristat etiprate) | Ipsen Pharma | Treatment of carcinoid syndrome diarrhoea in combination with somatostatin analogue (SSA) therapy in adults inadequately controlled by SSA therapy 09/2027 | |
| 175 | 147 | **Lutathera** (lutetium | Advanced Accelerator | Lutathera is indicated for the treatment of unresectable or metastatic, | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| | | (177Lu) oxodotreotide) | Applications | progressive, well differentiated (G1 and G2), somatostatin receptor positive gastroenteropancreatic neuroendocrine tumours (GEP NETs) in adults 09/2027 | |
| 176 | 148 | **Zejula**® (niraparib) | Tesaro UK limited | Zejula is indicated as monotherapy for the maintenance treatment of adult patients with platinum sensitive relapsed high grade serous epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete or partial) to platinum based chemotherapy 11/2027 | |
| 177 | 149 | **Chenodeoxycholic acid Leadiant** (chenodeoxycholic acid) | Leadiant GmbH | Treatment of inborn errors of primary bile acid synthesis due to sterol 27 hydroxylase deficiency (presenting as cerebrotendinous xanthomatosis (CTX)) in infants, **children** and adolescents aged 1 month to 18 years and adults 12/2027 | |
| **2018** | | | | | |
| 178 | 150 | **Jorveza**® (budesonide) | Dr. Falk Pharma GmbH | Eosinophilic esophagitis (EoE) in adults 1/2028 | |
| 179 | 151 | **Prevymis**® (letermovir) | Merck Sharp & Dohme Limited | Prophylaxis of cytomegalovirus (CMV) reactivation and disease in adult CMV-seropositive recipients [R+] of an allogeneic haematopoietic stem cell transplant (HSCT) 1/2028 | |
| 180 | 152 | **Crysvita**® (burosumab) | Kyowa Kirin Limited | X-linked hypophosphataemia (to be used in **children** over 1 year of age and adolescents) 3/2028 | |
| 181 | 153 | **Lamzede**® (velmanase alfa) | Chiesi Farmaceutici S.p.A. | Enzyme replacement therapy for the treatment of non-neurological manifestations in patients with mild to moderate alpha-mannosidosis (to be used in **children** aged 6 years and older, and adults) 3/2028 | |
| 182 | 154 | **Alofisel**® (darvadstrocel) | Tigenix, S.A.U. | Complex perianal fistulas in adults with Crohn's disease 4/2028 | |
| 183 | 155 | **Mylotarg**® (gemtuzumab ozogamicin) | Pfizer Limited | Indicated for combination therapy with daunorubicin (DNR) and cytarabine (AraC) for the treatment of patients age 15 years and above with previously untreated, de novo CD33 positive acute myeloid leukaemia (AML), except acute promyelocytic leukaemia (APL) 4/2028 | |
| 184 | 156 | **Amglidia**® (glibenclamide) | Ammtek | Neonatal diabetes mellitus **(newborns, infants and children)** 5/2028 | |
| 185 | 157 | **Rubraca**® (rucaparib) **WITHDRAWN and waiting for EC decision for a new indication** | Clovis Oncology UK Limited | High-grade cancers of the ovary, fallopian tubes and peritoneum. New indication 2018_ maintenance treatment of adult patients with platinum-sensitive relapsed high-grade epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete or partial) to platinum-based chemotherapy | 2018? |
| 186 | 158 | **Verkazia**® (ciclosporin) | Santen Oy | Treatment of severe vernal keratoconjunctivitis (VKC) in **children** from 4 years of age and adolescents | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| | | | | 7/2030 | |
| 187 | 159 | **Tegsedi®** (inotersen sodium) | Akcea Therapeutics Ireland Ltd | Treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR) 07/2028 | |
| 188 | 160 | **Myalepta®** (metreleptin) | Aegerion Pharmaceutic als B.V. | Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with or acquired partial LD (**Barraquer-Simons syndrome**), in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control 08/2028 | |
| 189 | | **Myalepta®** (metreleptin) | Aegerion Pharmaceutic als B.V. | Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with confirmed congenital generalised LD (**Berardinelli-Seip syndrome**) 08/2028 | |
| 190 | | **Myalepta®** (metreleptin) | Aegerion Pharmaceutic als B.V. | Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with or acquired generalised LD (**Lawrence syndrome**) in adults and children 2 years of age and above 08/2028 | |
| 191 | | **Myalepta®** (metreleptin) | Aegerion Pharmaceutic als B.V. | Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with confirmed **familial partial LD** 08/2028 | |
| 192 | 161 | **Vyxeos®** (daunorubicin / cytarabine) | Jazz Pharmaceutic als Ireland Limited | Adults with newly diagnosed, t herapy-related acute myeloid leukaemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC) 08/2028 | |
| 193 | 162 | **Mepsevii®** (vestronidase alfametreleptin) | Ultragenyx Germany GmbH | Non-neurological manifestations Mucopolysaccharidosis type VII (MPS VII, also known as Sly syndrome) 08/2028 | |
| 194 | 163 | **Kymriah®** (tisagenlecleucel) | Novartis Europharm Limited | Kymriah is indicated for the treatment of **paediatric** and young adult patients up to 25 years of age with B-cell acute lymphoblastic leukaemia (ALL) 8/2028 | |
| 195 | | **Kymriah®** (tisagenlecleucel) | Novartis Europharm Limited | Adult patients with relapsed or refractory diffuse large B cell lymphoma (DLBCL) 08/2028 | |
| 196 | 164 | **Yescarta®** (axicabtagene ciloleucel) | Kite Pharma EU B.V. | Treatment of adult primary mediastinal large B-cell lymphoma (PMBCL), after two or more lines of systemic therapy.lymphoma (PMBCL) 08/2028 | |
| 197 | | **Yescarta®** (axicabtagene ciloleucel) | Kite Pharma EU B.V. | Treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) 08/2028 | |
| 198 | 165 | **Onpattro®** | Alnylam Netherlands | Hereditary transthyretin-mediated amyloidosis (hATTR) in adult patients | |

| N° CHMP + opinions*a* | N° products*b* | Medicinal Product | MA Sponsor | Authorised Therapeutic Indication | New indic |
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| | | (patisiran) | B.V. | with stage 1 or stage 2 polyneuropathy 08/2028 | |
| 199 | 166 | **Cablivi®** (caplacizumab) | Ablynx NV | Adults experiencing an episode of acquired thrombotic thrombocytopenic purpura (aTTP), in conjunction with plasma exchange and immunosuppression 09/2028 | |
| 200 | 167 | **Symkevi®** (patisiran) | Vertex Pharmaceuticals (Europe) Ltd | Cystic fibrosis in patients aged **12 years** and above 11/2028 | |
| 201 | 168 | **Takhzyro** (lanadelumab) | Shire Pharmaceuticals Ireland Limited | Indicated for routine prevention of recurrent attacks of hereditary angioedema (HAE) in patients aged **12 years and older** 11/2028 | |
| 202 | 169 | **Poteligeo** (Mogamulizumab) | Kyowa Kirin Holdings B.V. | Treatment of adult patients with mycosis fungoides (MF) or Sézary syndrome (SS) who have received at least one prior systemic therapy 11/2028 | |
| 203 | 170 | **Luxturna®** (voretigene neparvovec) | Spark Therapeutics Ireland Ltd | Adults and **children** with loss of vision due to inherited retinal dystrophies 1) Leber's congenital amaurosis 2) retinitis pigmentosa 12/2028 | |
| 204 | 171 | **Namuscla®** (mexiletine hcl) | Lupin Europe GmbH | Symptomatic treatment of myotonia in adult patients with non-dystrophic myotonic disorders 12/2028 | |
| **2019** | | | | | |
| 205 | 172 | **Waylivra®** (volanesorsen) | Akcea Therapeutics Ireland Limited | Hyperlipoproteinemia Type I | |
| 206 | 173 | **Zynteglo®** (Autologous CD34+ cells encoding βA-T87Q-globin gene) | bluebird bio (Netherlands) B.V. | beta-Thalassemia | |

***a*** = Number of positive CHMP opinions; ***b*** =Number of *different* products ***c*** = International Non-proprietary Name (INN)

**EXPIRED** product reached the end of the period of market exclusivity

**WITHDRAWN:** withdrawn from the Community Register of orphan medicinal products upon request of the marketing authorisation holder

**WITHDRAWN FROM THE MARKET – SAFETY** withdrawn from the market in the European Union due to safety reasons

**XX/XXXX** Date that the market exclusivity will end

Executive Agency for Health and Consumers

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