

Nye legemidler som ikke har markedsføringstillatelse

For legemidler oppført på listen gjelder retningslinjens vilkår uavhengig av indikasjon for bruken.

Oppdatert: 21.03.2025

| Virkestoff | Indikasjon per nå. (Samt mulig andre indikasjoner i fremtiden.) | Orphan medicinal product (Se informasjon om COMP (Komiteen for legemidler mot sjeldne sykdommer) på www.legemiddelverket.no) | Oppført på listen |
|--|---|--|-------------------|
| abicipar pegol | Treatment of neovascular (wet) age related macular degeneration (AMD) | | aug.19 |
| acalabrutinib | Treatment of mantel cell lymphoma - Kun denne indikasjonen | | mar.18 |
| aficamten | Treatment of symptomatic obstructive hypertrophic cardiomyopathy (oHCM) in adult patients | | jan.25 |
| allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded | Treatment of graft-versus-host disease. | x | sep.23 |
| alpelisib | Treatment of patients with severe manifestations of PIK3CArelated overgrowth spectrum - Kun denne indikasjonen | x | aug.22 |
| apadamtase alfa | Treatment of congenital thrombotic thrombocytopenic purpura (cTTP) due to ADAMTS13 deficiency | x | jun.23 |
| apitegromab | Treatment of spinal muscular atrophy | | mar.25 |
| arimoclomol | Treatment of Niemann-Pick disease type C (NPC) | x | des.20 |
| artesunate (*Kun styrken 60 mg) | Treatment of severe malaria | x | feb.21 |
| aumolertinib | Treatment of adult patients with locally advanced or metastatic non-small cell lung cancer | | des.22 |
| autologous human chondrocytes in vitro expanded | Repair of cartilage defects of the knee joint | | sep.20 |

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| autologous cartilage-derived articular chondrocytes, in-vitro expanded | Repair of symptomatic, localised, full-thickness cartilage defects of the knee joint grade III or IV | | jan.24 |
| autologous glioma tumor cells, inactivated / autologous glioma tumor cell lysates, inactivated / allogeneic glioma tumor cells, inactivated / allogeneic glioma tumor cell lysates, inactivated | Treatment of glioma | x | okt.20 |
| Autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding human Wiskott-Aldrich syndrome protein | Treatment of patients with Wiskott-Aldrich Syndrome (WAS) | x | jan.25 |
| bardoxolone methyl | Treatment of chronic kidney disease (CKD) | x | okt.21 /des.21 |
| belantamab mafodin | Treatment of multiple myeloma | | aug.24 |
| belinostat | Relapserad/refraktärt T-cellslymfom | | mar.24 |
| belumosudil | Treatment of chronic graft-versushost disease (cGVHD) after failure of at least two prior lines of systemic therapy/ Graft versus host disease (GvHD) | x | okt. 24/ nov. 22 |
| beremagene-geperpavec | Treatment of patients from birth with dystrophic epidermolysis bullosa (DEB) with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene. Juni 2023: Treatment of wounds in patients with dystrophic epidermolysis bullosa (DEB) | x | des.23 /jun.23 |
| bifikafusp alfa /onfekafusp alfa | Neoadjuvant treatment of adult patients with locally advanced fully resectable melanoma | | jul.24 |
| blarcamesine | Treatment of Alzheimer's disease and dementia | | jan.25 |
| casimersen | Treatment of duchennes muscular dystrophy | | okt.24 |
| copanlisib | Treatment of non-hodgkin lymphoma | | mar.18 |
| copanlisib | Treatment of adult patients with marginal zone lymphoma | x | jul.22 |
| daprodustat | Treatment of anemia associated with chronic kidney disease (CKD) in adults | | apr.22 |
| datopotamab | Treatment of adult patients with locally advanced or metastatic non squamous nonsmall cell lung cancer (NSCLC) | | apr.24 |

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| deferiprone | Treatment of neurodegeneration with brain iron accumulation - Kun denne indikasjonen | x | jun.19 |
| delandistrogene moxeparvovec | Treatment of ambulatory patients aged 3 to 7 years old with Duchenne muscular dystrop. Treatment of Duchenne muscular dystrophy | x | jul.24/ aug.23 |
| depatuximab mafodotin | Treatment of glioblastoma (GBM) | | nov.18 |
| deutivacaftor / tezacaftor /vanzacaftor Orphan | Indicated for the treatment of cystic fibrosis | | jun.24 |
| diclofenamide | Treatment of periodic paralysis | | feb.19 |
| Diflunisal | Treatment of ATTR amyloidosis | x | mar.24 |
| Donanemab | To slow disease progression in adult patients with Alzheimer's disease (AD) | | mai.24 |
| donidalorsen | For routine prevention of recurrent attacks of hereditary angioedema (HAE) | | jan.25 |
| dorocubice / allogeneic umbilical cord-derived CD34- cells, non-expanded | Treatment of adult patients with haematological malignancies | x | jul.24 |
| doxetine / doxribtimine - | Indicated for the treatment of paediatric and adult patients with thymidine kinase 2 deficiency (TK2d) with an age of symptom onset on or before 12 years | | des.24 |
| duvelisib | Treatment of adult patients with small lymphocytic lymphoma (SLL) - Kun denne indikasjonen | x | feb.20 |
| edaravone | Amyotrophic lateral sclerosis (ALS) | | jan.18 |
| eflornithine / sulindac | Treatment of familial adenomatous polyposis | x | jul.20 |
| eflornithine | Treatment of high-risk neuroblastoma responsive to prior multiagent, multimodality therapy - Kun denne indikasjonen | x | jan.25 |
| elinzanetant | for the treatment of moderate to severe vasomotor symptoms (VMS) | | des.24 |
| enasidenib | Treatment of acute myeloid leukaemia | | mar.18 |
| entolimod | Treatment of acute radiation syndrome | x | nov.17 |
| eteplirsen | Treatment of duchennes muscular dystrophy | | okt.24 |
| ferric citrate coordination complex | Treatment of iron deficiency anaemia in adult chronic kidney disease (CKD) patients with elevated serum phosphorus levels | | jun.24 |
| ferumoxytol | Intravenous treatment of iron deficiency anaemia (IDA) | | jun.22 |
| glutamine | Treatment of sickle cell disease | | mar.18 |

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| golodirsen | Treatment of duchennes muscular dystrophy | | okt.24 |
| govorestat | Treatment of adults and children aged 2 years and older with a confirmed diagnosis of classic galactosemia | x | jan.24 |
| idebenone | Treatment of respiratory dysfunction in patients with Duchenne muscular dystrophy (DMD) not using glucocorticoids - Kun denne indikasjonen | x | aug.19 |
| imlunestrant | Monotherapy and in Combination with Verzenio® (abemaciclib) in Patients with ER+, HER2- Advanced Breast Cancer | | jan.25 |
| inavolisib | Treatment of adult patients with PIK3CA-mutated, hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative, locally advanced or metastatic breast cancer | | jun.24 |
| infigratinib | Treatment of cholangiocarcinoma | x | des.21 |
| ipatasertib | Treatment of breastcancer | | mar.18 |
| l-acetylleucine | indicated in adults and children from birth for chronic treatment of Niemann-Pick Type C (NPC) | | jul.24 |
| lecanemab | Indicated as a disease modifying treatment in adult patients with Mild Cognitive Impairment due to Alzheimer's disease and Mild Alzheimer's disease (Early Alzheimer's disease) | | feb.23 |
| lenadogene nolparvovec | Treatment of vision loss due to Leber Hereditary Optic Neuropathy (LHON) | x | nov.20 |
| lenacapavir | Pre-exposure prophylaxis to prevent HIV-1 (kun denne indikasjonen) | | mar.25 |
| leniolisib | Treatment of activated phosphoinositide 3-kinase delta syndrome (APDS) | x | nov.22 |
| lifileucel | Treatment of unresectable or metastatic melanoma | | sep.24 |
| linvoseltamab | Monotherapy for the treatment of adult patients with relapsed or refractory multiple myeloma | | mar.24 |
| LUT014 | Topical BRAF Inhibitor Therapy for EGFR Inhibitor–Induced Acneiform Rash | | nov.23 |

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| mavoxifafor | Treatment of WHIM syndrome | x | jan.25 |
| mirdametininib | Treatment of neurofibromatosis type 1 | | sep.24 |
| mobocertininib | Treatment of adult patients with epidermal growth factor receptor (EGFR) exon 20 insertion mutation-positive locally advanced or metastatic non-small cell lung cancer (NSCLC). | | jan.21 |
| mozafancogene autotemcel | Treatment of paediatric patients with Fanconi Anaemia Type A | x | mai.24 |
| nadofaragene firadenovec | Treatment of adult patients with high-grade(HG), Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladdercancer (NMIBC)., | | jan.25 |
| naporafeninib | Treatment of advanced solid tumours including ovarian cancer | | okt.21 |
| nedosiran | Til behandling av primær hyperoksaluri. | | mai.24 |
| nerandomilast | Treatment of idiopathic pulmonary fibrosis. Pulmonary fibrosis | | mar.25 |
| nipocalimab | treatment of generalised Myasthenia Gravis | | okt.24 |
| nemolizumab | Treatment of moderate-to-severe atopic dermatitis and for the treatment of prurigo nodularis | | mar.24 |
| nirogacestat | Treatment of desmoid tumours | x | apr.24 |
| nogapendekin alfa / Inbakicept | Treatment of adult patients with BCG-unresponsive non-muscle invasive bladdercancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumours | | jan.25 |
| obecabtagene autoleucel | Treatment of patients with relapsed or refractory B cell precursor acute lymphoblastic leukaemia (ALL) | x | mai.24 |
| obeticholic acid | Improvement of liver fibrosis and resolution of steatohepatitis in adult patients with significant liver fibrosis due to nonalcoholic steatohepatitis (NASH). Kun denne indikasjonen | | aug.20 |
| obnitix | Treatment of RR-aGVHD | | apr.24 |
| odevixibat | Treatment of cholestatic pruritus in Alagille syndrome (ALGS) Kun denne indikasjonen | | jan.24 |
| olezarsen | Treatment of familial chylomicronemia | | sep.24 |

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| omadacycline tosylate | Treatment of community-acquired bacterial pneumonia (CABP) and acute bacterial skin and skin structure infections (ABSSSI) in adults | | okt.18 |
| omecamtiv mecarbil | Treatment of adult patients with symptomatic chronic heart failure and reduced ejection fraction less than 30% | | feb.23 |
| oportuzumab monatox | Treatment and prevention of recurrence of carcinoma-in-situ (CIS) of the urinary bladder and prevention of recurrence of high grade Ta and/or T1 papillary tumours | | apr.21 |
| paltusotine | Treatment of acromegaly | x | mar.25 |
| parsaclisib | Treatment of adult patients with relapsed or refractory marginal zone lymphoma (MZL) | x | feb.22 |
| pegcetacoplan | Treatment of geographic atrophy (GA) secondary to age-related macular degeneration (AMD) - Kun denne indikasjonen | x | feb.23 |
| pevonedistat | Higher-risk chronic myelomonocytic leukemia (HR-CMML), low-blast acute myeloid leukemia (LB-AML) and higher risk myelodysplastic syndromes | | aug.20 |
| plazomicin | Treatment of symptomatic tenosynovial giant cell tumor (TGCT), also known as pigmented villonodular synovitis (PVNS) and giant cell tumor of the tendon sheath (GCT-TS), where surgical resection is potentially associated with worsening functional limitation or severe morbidity | x | mar.19 |
| plazomicin | Treatment of Complicated urinary tract infection (cUTI), including pyelonephritis; treatment of Bloodstream infection (BSI); treatment of infections due to Enterobacteriaceae | | nov.18 |
| plozasiran | Treatment of familial chylomicronemia syndrome | | mar.25 |
| pridopidine | Treatment of Huntington's disease | x | sep.24 |
| remibrutinib | Treatment of chronic spontaneous urticaria. Treatment of chronic inducible urticaria | | mar.25 |
| resmetirom | Treatment of adults with nonalcoholic steatohepatitis (NASH)/metabolic dysfunction associated steatohepatitis (MASH) with liver fibrosis | | apr.24 |

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| resminostat | Treatment of patients with advanced stage mycosis fungoides (MF) and Sézary syndrome (SS) | | apr.24 |
| retifanlimab | Treatment of locally advanced or metastatic squamous carcinoma of the anal canal (SCAC) who have progressed on or who are intolerant of platinum (kun denne indikasjonen) | x | mar.21 |
| rilonacept | treatment of idiopathic pericarditis | x | okt.24 |
| rilzabrutinib | Treatment of persistent or chronic immune thrombocytopenia (ITP) | x | des.24 |
| romidepsin | Treatment of peripheral T-cell lymphoma (PTCL) in adult patients who have received at least one prior therapy. | | feb.22 |
| rovalpituzumab tesirine | Small cell lung cancer (SCLC) | | jan.18 |
| savolitinib | monotherapy for the treatment of adult patients with relapsed or refractory multiple myeloma | | mar.23 |
| sebetralstat | treatment of hereditary angioedema (HAE) attacks in adult and adolescents aged 12 years and older. | x | sep.24 |
| sepiapterin | Treatment of hyperphenylalaninemia (HPA) in adult and paediatric patients with phenylketonuria (PKU) patients with phenylketonuria (PKU). | | jun.24 |
| siponimod | Indicated for the treatment of thrombocytopenia - Kun denne indikasjonen | | okt.18 |
| sodium phenylbutyrate/ ursodoxicoltaurine | Treatment of amyotrophic lateral sclerosis (ALS) | x | apr.22 |
| surufatinib | Treatment of progressive neuroendocrine tumours | | jul.21 |
| tazemetostat | Treatment of malignant mesothelioma and Epithelioid sarcoma | | mai.21 |
| tegomil fumarat | Treatment of multiple sclerosis | | mai.24 |
| teplizumab | To delay both the onset of Stage 3 type 1 diabetes (T1D) and the progression of Stage 3 T1D. Utsette klinisk debut av type 1 diabetes | | jan.25/ jul.24 |
| teprotumumab | Treatment of moderate to severe Thyroid Eye Disease (TED) | | jun.24 |
| tisotumab vedotin | treatment of adult patients with recurrent or metastatic cervical cancer with disease progression on or after systemic therapy | | mar.24 |
| tovorafenib | Treatment of paediatric low grade glioma | | mar.25 |

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| trastuzumabduocarmazine | Treatment of HER2 (Human Epidermal Growth Factor Receptor 2)-positive metastatic breast | | aug.22 |
| trofinetide | Treatment of Rett syndrome in adults and paediatric patients 2 years of age and older | x | jan.25 |
| troriluzole | Treatment of adult patients with spinocerebellar ataxia genotype 3 (SCA3 | x | nov.23 |
| valemetostat | Ved tilbakevendende/refraktær (R/R) adult T-cell leukemia/lymphoma (ATLL). | | apr.22 |
| veliparib | Treatment of BRCA 1, BRCA 2, BARD1 and/orr PALB2 mutated cancer | | mar.18 |
| viltolarsen | Treatment of duchennes muscular dystrophy | | okt.24 |
| vimseltinib | Treatment of adult patients with tenosynovial giant cell tumour (TGCT) who are not amenable to surgery | x | aug.24 |
| Vorasidenib | Til behandling av gliom | | mai.24 |
| zanidatamab | Treatment of biliary tract cancer | x | jul.24 |