

## Nye legemidler som ikke har markedsføringstillatelse

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

Oppdatert: 24.11.2020

Virkestoff	Indikasjon per nå. (Samt mulig andre indikasjoner i fremtiden.)	Orphan medicinal product (Se informasjon om COMP (Komiteen for legemidler mot sjeldne sykdommer) på <a href="http://www.legemiddelverket.no">www.legemiddelverket.no</a> )	Oppført på listen
Abicipar pegol	Treatment of neovascular (wet) age-related macular degeneration (AMD)		aug.19
Abrocitinib	Indicated for the treatment of moderate-to-severe atopic dermatitis in adults and adolescents 12 years and older who are candidates for systemic therapy.		okt.20
Acalabrutinib	Treatment of mantel cell lymphoma ( <b>kun denne indikasjonen</b> )		mar.18
Aducanumab	Alzheimer's disease		nov.20
Anifrolumab	Indicated as an add-on therapy for the treatment of adult patients with moderate to severe systemic lupus erythematosus (SLE), despite standard therapy		nov.20
Arachis hypogaea allergens	Immunotherapy (OIT) for patients aged 4 to 17 years with a confirmed diagnosis of peanut allergy		aug.19
Asciminib	Treatment for acute myeloid leukaemia (AML) and acute lymphoblastic leukemia (ALL)		des.19
Autologous cd34+ cell enriched population that contains hematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector encoding the human arylsulfatase a gene	Treatment of metachromatic leukodystrophy (MLD)	x	des.19
Autologous human chondrocytes in vitro expanded	Repair of cartilage defects of the knee joint		sep.20

Autologous peripheral blood t cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured	Treatment of adult patients with relapsed or refractory Mantle cell lymphoma (MCL)	x	aug.20
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
Avacopan	Treatment of granulomatosis with polyangiitis (GPA) or microscopic polyangiitis (MPA)	x	nov.20
Avalglucosidase alfa	For long-term enzyme replacement therapy for the treatment of patients with Pompe disease	x	okt.20
Avapritinib	Treatment of mastocytosis ( <b>Kun denne indikasjonen</b> )		mar.18
Azacitinidine	Treatment for acute myeloid leukaemia <b>* Gjelder kun andre formuleringer enn pulver til injeksjonsvæske, suspensjon.</b>		jun.20
Berotrastat	Prevention of hereditary angioedema (HAE)	x	apr.20
Bimekizumab	Treatment of plaque psoriasis		sep.20
Copanlisib	Treatment of non-hodgkin lymphoma		mar.18
Crizanlizumab	Treatment of sickle cell disease	x	aug.19
Deferiprone	Treatment of neurodegeneration with brain iron accumulation ( <b>Kun denne indikasjonen</b> )	x	jun.19
Depatuximab mafodotin	Treatment of glioblastoma (GBM)		nov.18
Diclofenamide	Treatment of periodic paralysis		feb.19
Dostarlimab	Treatment of mismatch repair deficient (dMMR)/microsatellite instability-high (MSI-H) endometrial cancer (EC)		apr.20
Duvelisib	Treatment of adult patients with relapsed or refractory chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL) and relapsed or refractory follicular lymphoma (FL)	x	feb.20
Edaravone	Amyotrophic lateral sclerosis (ALS)		jan.18

Eflornithine / sulindac	Treatment of familial adenomatous polyposis	x	jul.20
Eladocagene exuparvovec	Treatment of aromatic L-amino acid decarboxylase (AADC) deficiency	x	mar.20
Elivaldogene autotemcel	Treatment of ABCD1 genetic mutation and cerebral adrenoleukodystrophy	x	okt.20
Enasidenib	Treatment of acute myeloid leukaemia		mar.18
Entolimod	Treatment of acute radiation syndrome	x	nov.17
Erdafitinib	Treatment of adults with locally advanced or metastatic urothelial carcinoma		nov.19
Evinacumab	Treatment of Homozygous Familial Hypercholesterolemia (HoFH)		okt.20
fedratinib	Treatment of primary myelofibrosis, post polycythaemia vera myelofibrosis or post essential thrombocythaemia myelofibrosis	x	aug.20
Fenfluramine	Treatment of seizures associated with Dravet syndrome in children aged 2 years to 17 years and adults.	x	mar.19
Glutamine	Treatment of sickle cell disease		mar.18
Glucarpidase	Treatment of patients at risk of methotrexate toxicity	x	sep.20
Hydrocortison	Replacement therapy for congenital adrenal hyperplasia (CAH) in adolescents aged 12 years and over and adults ( <b>Kun denne indikasjonen</b> )	x	apr.20
Idebenone	Treatment of respiratory dysfunction in patients with Duchenne muscular dystrophy (DMD) not using glucocorticoids ( <b>Kun denne indikasjonen</b> )	x	aug.19
Idecabtagene vicleucel	Treatment of multiple myeloma	x	jun.20
Inclisiran	Treatment for primary hypercholesterolaemia or mixed dyslipidaemia		aug.20
Ipatasertib	Treatment of breastcancer		mar.18
Ivosidenib	Treatment of adult patients (≥ 18 years old) with relapsed or refractory acute myeloid leukaemia (AML) with an isocitrate dehydrogenase-1 (IDH1) R132 mutation	x	feb.19
Lenadogene nolparvovec	Treatment of vision loss due to Leber Hereditary Optic Neuropathy (LHON)	x	nov.20

Lisocabtagene maraleucel	Treatment of large B-cell lymphoma, diffuse large B-cell lymphoma (DLBCL), primary mediastinal large B-cell lymphoma (PMBCL) and follicular lymphoma grade 3B (FL3B)	x	aug.20
Lonafarnib	Treatment of Hutchinson-Gilford Progeria Syndrome and Progeroid Laminopathies	x	mai.20
Lonapegsomatropin	Treatment of growth hormone deficiency	x	okt.20
Moxetumomab pasudotox	Relapsed or refractory hairy cell leukaemia (HCL) after receiving at least two prior systemic therapies	x	feb.20
Obiltoximab	Treatment of inhalational anthrax due to Bacillus anthracis	x	aug.19
Obeticholic acid	improvement of liver fibrosis and resolution of steatohepatitis in adult patients with significant liver fibrosis due to nonalcoholic steatohepatitis (NASH) <b>Kun denne indikasjonen</b>		aug.20
Ofatumumab	Treatment of relapsing forms of multiple sclerosis <b>(Kun denne indikasjonen)</b>		aug.20
Olipudase alfa	Treatment of non-neurological manifestations of acid sphingomyelinase deficiency		okt.18
Omadacycline tosylate	Treatment of community-acquired bacterial pneumonia (CABP) and acute bacterial skin and skin structure infections (ABSSSI) in adults		okt.18
Pegcetacoplan	Paroxysmal nocturnal haemoglobinuria (PNH)	x	okt.20
Pemigatinib	Treatment of locally advanced or metastatic cholangiocarcinoma		feb.20
Pertuzumab / trastuzumab <b>(Kun denne kombinasjonen, ikke løs kombo)</b>	Treatment of early breast cancer, metastatic breast cancer		aug.20
Pevonedistat	Higher-risk chronic myelomonocytic leukemia (HR-CMML), low-blast acute myeloid leukemia (LB-AML) and higher-		aug.20

Pexidartinib	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);		nov.18
Ponesimod	Treatment of adult patients with relapsing forms of multiple sclerosis (RMS) with active disease defined by		apr.20
Pralsetinib	Treatment of non-small cell lung cancer (NSCLC)		jun.20
Quizartinib	Treatment for acute myeloid leukaemia	x	nov.18
Ranibizumab	Treatment of neovascular age-related macular degeneration (AMD)		okt.20
Remimazolam	Indicated for procedural sedation		feb.20
Ripretinib	Treatment of patients with advanced gastrointestinal stromal tumour (GIST)		jan.20
Risdiplam	Treatment of spinal muscular atrophy (SMA)	x	mai.20
Roxadustat	Treatment of anaemia		jun.20
Rovalpituzumab tesirine	Small cell lung cancer (SCLC)		jan.18
Satralizuma	Treatment of adult and adolescent patients from 12 years of age with neuromyelitis optica spectrum disorders (NMOSD)	x	okt.19
Selinexor	Treatment of patients with relapsed refractory multiple myeloma (RRMM)	x	jan.19

Selpercatinib	Indicated for the treatment of adults with: advanced RET fusion-positive non-small cell lung cancer (NSCLC) who require systemic therapy; advanced RET fusion-positive thyroid cancer who require systemic therapy and who have progressed following prior treatment. As monotherapy is indicated for the treatment of adults and adolescents 12 years and older with advanced RET-mutant medullary thyroid cancer( MTC) who require systemic therapy		aug.20
Selumetinib	Treatment of neurofibromatosis type 1 (NF1)	x	mar.18, apr.20
Setmelanotide	Treatment of obesity and the control of hunger associated with deficiencies in	x	aug.20
Siponimod	Indicated for the treatment of thrombocytopenia ( <b>* kun denne indikasjonen</b> )		okt.18
Somapacitan	Indicated for the replacement of endogenous GH with growth hormone deficiency (AGHD)	x	okt.19
Tafasitamab	Indicated in combination with lenalidomide followed by Tafasimab monotherapy for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL), including DLBCL arising from low grade lymphoma, who are not eligible for, or refuse, autologous stem cell transplant (ASCT)	x	jun.20
Tagraxofusp	Treatment of adult patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN)	x	feb.19
Talazoparib	Treatment of prostatecancer ( <b>kun denne indikasjonen</b> )		mar.18
Tecovirimat	Treatment of orthopoxvirus disease		nov.20
<b>Trastuzumab Deruxtecan (Kun dette virkestoffet)</b>	Treatment of HER2 Positive Metastatic Breast Cancer		jul.20
Tralokinumab	Treatment of moderate-to-severe atopic dermatitis in adult patients who are candidates for systemic therapy		jun.20

Tukatinib	Treatment of metastatic breast cancer or brain metastases		aug.20
Valoctocogene roxaparvovec	Treatment of haemophilia A	x	feb.20
Veliparib	Treatment of BRCA 1, BRCA 2, BARD1 and/or PALB2 mutated cancer		mar.18
Vericiguat	Treatment of symptomatic chronic heart failure		jul.20
Vosoritide	Indicated for the treatment of achondroplasia	x	sep.20
Zanubrutinib	Treatment of Waldenström's macroglobulinaemia (WM)	x	jul.20