

## Nye legemidler som ikke har markedsføringstillatelse

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

Oppdatert: 02.03.2020

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
Acalabrutinib	Treatment of mantel cell lymphoma		mar.18
Alpelisib	Treatment of with hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative, advanced breast cancer with a PIK3CA mutation in combination with fulvestrant after disease progression following an endocrine-based regimen		okt.18
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
Avapritinib	Treatment of mastocytosis and GIST		mar.18
Brolucizumab	Treatment of neovascular (wet) age-related macular degeneration (AMD)		mar.19

Cefiderocol	Treatment of infections caused by carbapenem-resistant Gram-negative bacteria in adult patients with limited treatment options. Treatment of infections caused by aerobic Gram-negative bacteria in adult patients with limited treatment options		jan.19
Copanlisib	Treatment of non-hodgkin lymphoma		mar.18
Crizanlizumab	Treatment of sickle cell disease	x	aug.19
Darolutamide	Non-metastatic castration resistant prostate cancer		mar.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
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
Eladocagene exuparvovec	Treatment of aromatic L-amino acid decarboxylase (AADC) deficiency	x	mar.20
Enasidenib	Treatment of acute myeloid leukaemia		mar.18
Entolimod	Treatment of acute radiation syndrome	x	nov.17
Entrectinib	Treatment of adult and paediatric patients with neurotrophic tyrosine receptor kinase (NTRK) fusion-positive locally advanced or metastatic solid tumours. Treatment of patients with ROS1-positive, advanced non-small cell lung cancer (NSCLC)		jan.19
Erdafitinib	Treatment of adults with locally advanced or metastatic urothelial carcinoma		nov.19
Fenfluramine	Treatment of seizures associated with Dravet syndrome in children aged 2 years to 17 years and adults.	x	mar.19
Fostamatinib	Indicated for the treatment of thrombocytopenia		okt.18

Givosiran	Treatment of acute hepatic porphyria (AHP) in adults and adolescents aged 12 years and older	x	aug.19
Glasdegib	Treatment of newly diagnosed de novo or secondary acute myeloid leukaemia	x	jun.19
Glutamine	Treatment of sickle cell disease		mar.18
Idebenone	Treatment of respiratory dysfunction in patients with Duchenne muscular dystrophy (DMD) not using glucocorticoids <b>(Kun denne indikasjonen)</b>	x	aug.19
Imipenem/ Cilastatin/ Relebactam	Treatment of bacterial infections due to gram-negative microorganisms		jan.19
Imlifidase	Indicated for desensitization treatment of highly sensitized adult kidney transplant patients with positive crossmatch against an available deceased donor	x	mar.19
Ipatasertib	Treatment of breastcancer		mar.18
Isatuximab	Treatment of patients with multiple myeloma (MM)	x	jun.19
Ivosidenib	Treatment of adult patients ( $\geq 18$ years old) with relapsed or refractory acute myeloid leukaemia (AML) with an isocitrate dehydrogenase-1 (IDH1) R132 mutation	x	feb.19
Lefamulin	Treatment of community-acquired pneumonia (CAP)	x	aug.19
Luspatercept	Treatment of adult patients with very low to intermediate-risk myelodysplastic syndromes (MDS)-associated anaemia; - the treatment of adult patients with beta-thalassaemia ( $\beta$ -thalassaemia)-associated anaemia who require RBC transfusions	x	jun.19
Methylthioninium chloride	Indicated as an aid for the enhanced visualization and detection of colorectal lesions in adult patients undergoing screening / surveillance colonoscopy for colorectal cancer. An increased detection of colorectal lesions translates into an increase in the adenoma detection rate (ADR). (kun denne spesifikke		mar.19
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
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
Onasemnogene abeparvovec	Treatment of spinal muscular atrophy (SMA)	x	nov.18
Osilodrostat	Treatment of Cushing's syndrome	x	jan.19
Pemigatinib	Treatment of locally advanced or metastatic cholangiocarcinoma		feb.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
Quizartinib	Treatment for acute myeloid leukaemia	x	nov.18
Remimazolam	Indicated for procedural sedation		feb.20
Ripretinib	GIST		jan.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
Selumetinib	Neurofibromatosis		mar.18
Siponimod	Indicated for the treatment of thrombocytopenia ( <b>* kun denne indikasjonen</b> )		okt.18
Somapacitan	Indicated for the replacement of	x	okt.19

Tagraxofusp	Treatment of adult patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN)	x	feb.19
Talazoparib	Treatment of prostatecancer ( <b>kun denne</b>		mar.18
Treprostinil sodium	Treatment of thromboembolic pulmonary hypertension (CTEPH) (kun denne spesifikke indikasjonen)	x	mar.19
Valoctocogene roxaparvovec	Treatment of haemophilia A	x	feb.20
Veliparib	Treatment of BRCA 1, BRCA 2, BARD1 and/or PALB2 mutated cancer		mar.18
VX-445/tezacaftor/Ivacaftor	Treatment of cystic fibrosis (CF)	x	okt.19