

Nye legemidler som ikke har markedsføringstillatelse

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

Oppdatert: 02.02.2023

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
Adagrasib	Treatment of patients with advanced non-small cell lung cancer (NSCLC) with KRAS G12C mutation		jun.22
Alpelisib	Treatment of patients with severe manifestations of PIK3CArelated overgrowth spectrum - Kun denne indikasjonen	x	aug.22
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
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
Bardoxolone methyl	Treatment of chronic kidney disease (CKD)	x	okt.21 /des.21
Belumosudil	Graft versus host disease (GvHD)		nov.22

Bevacizumab	Treatment of neovascular (wet) age-related macular degeneration (nAMD) - Kun denne indikasjonen		feb.23
Cabotegravir	Pre-exposure prophylaxis of HIV-1 infection - Kun denne indikasjonen		nov.22
Cipaglucosidase alfa	Treatment of adults aged 18 years and older with a confirmed diagnosis of Pompe disease	x	des.21
Concizumab	indicated for routine prophylaxis to prevent or reduce the frequency of bleeding in patients with - haemophilia A (congenital factor VIII deficiency) with FVIII inhibitors \geq 12 years of age. - haemophilia B (congenital factor IX deficiency) with FIX inhibitors of any age.		feb.23
Copanlisib	Treatment of non-hodgkin lymphoma		mar.18
Copanlisib	Treatment of adult patients with marginal zone lymphoma	x	jul.21
Dabrafenib	Treatment of glioma - Kun denne indikasjonen	x	okt.22
Daprodustat	Treatment of anaemia associated with chronic kidney disease (CKD) in adults		apr.22
Deferiprone	Treatment of neurodegeneration with brain iron accumulation - Kun denne indikasjonen	x	jun.19
Depatuximab mafodotin	Treatment of glioblastoma (GBM)		nov.18
Deucravacitinib	Treatment of moderate to severe plaque psoriasis in adults who are candidates for systemic therapy		okt.21
Diclofenamide	Treatment of periodic paralysis		feb.19
Duvelisib	Treatment of adult patients with small lymphocytic lymphoma (SLL) - Kun denne indikasjonen	x	feb.20
Edaravone	Amyotrophic lateral sclerosis (ALS)		jan.18
Efbemalenograstim alfa	Reduction in the duration of neutropenia and the incidence of febrile neutropenia		nov.21
Eflornithine / sulindac	Treatment of familial adenomatous polyposis	x	jul.20
Elranatamab	Indicated as monotherapy for the treatment of relapsed or refractory multiple myeloma in adult patients	x	jan.23
Enasidenib	Treatment of acute myeloid leukaemia		mar.18
Entolimod	Treatment of acute radiation syndrome	x	nov.17

Epcoritamab	Treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL)	x	nov.22
Erdafitinib	Treatment of adults with locally advanced or metastatic urothelial carcinoma		nov.19
Etranacogene dezaparvove	Treatment of adults with Haemophilia B (congenital Factor IX deficiency) and with a preexisting neutralising anti-AAV5 antibody titre below 1:700 to reduce the frequency of bleeding episodes and the need for Factor IX replacement therapy		apr.22
Etracimod	Treatment of patients with moderately to severely active ulcerative colitis (UC)		des.22
Exagamglogene autotemcel	For sickle Cell Disease and Beta Thalassemia		jan.23
Ferumoxytol	Intravenous treatment of iron deficiency anaemia (IDA)		jun.22
Futibatinib	Treatment of cholangiocarcinoma		jun.22
Ganaxolone	treatment of epileptic seizures associated with cyclindependent kinase-like 5 deficiency disorder (CDD)		des.21
Gefapixant	Treatment of refractory or unexplained chronic cough		mar.21
Glofitamab	Relapsed or refractory (R/R) B-cell non-Hodgkin lymphoma (B-NHL), treatment of diffuse large B-cell lymphoma		mai.21
Glutamine	Treatment of sickle cell disease		mar.18
Idebenone	Treatment of respiratory dysfunction in patients with Duchenne muscular dystrophy (DMD) not using glucocorticoids - Kun denne indikasjonen	x	aug.19
Infigratinib	Treatment of cholangiocarcinoma	x	des.21
Ipatasertib	Treatment of breastcancer		mar.18
Ivosidenib	Treatment of acute myeloid leukaemia and treatment of metastatic cholangiocarcinoma. Treatment of adult patients (≥ 18 years old) with relapsed or refractory acute myeloid leukaemia (AML) with an isocitrate dehydrogenase-1 (IDH1) R132 mutation.	x	apr.22/ feb.19
Lebrikizumab	Treatment of moderate-to-severe atopic dermatitis in adults and adolescents		nov.22

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
Leniolisib	Treatment of activated phosphoinositide 3-kinase delta syndrome (APDS)	x	nov.22
Naporafenib	Treatment of advanced solid tumours including ovarian cancer		okt.21
Maralixibat	Treatment of cholestatic liver disease in patients with Alagille syndrome (ALGS) 1 year of age and older, Treatment of Progressive Familial Intrahepatic Cholestasis Type 2	x	nov.21, des.20
Mavacamten	Treatment of symptomatic obstructive hypertrophic cardiomyopathy		jul.21
Miglustat	treatment of adults aged 18 years and older with a confirmed diagnosis of Pompe disease Kun denne indikasjonen	x	des.21
Mirikizumab	Treatment of moderately to severely active ulcerative colitis		jun.22
Momelotinib	Treatment of disease-related splenomegaly or symptoms and anaemia	x	des.22
Mobocertinib	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
Niraparibtosylatemonohydrat / Abirateronacetate	Treatment of adult patients with prostate cancer		jun.22
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
Omadacycline tosylate	Treatment of community-acquired bacterial pneumonia (CABP) and acute bacterial skin and skin structure infections (ABSSSI) in adults		okt.18
Omaveloxolone	Treatment of Friedreich's ataxia	x	feb.23
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
Palopegteriparatid	Replacement therapy indicated for the treatment of hypoparathyroidism in adults	x	jan.23
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		feb.23
Pegunigalsidase alfa	Treatment of Fabry disease	x	apr.22
Pevedistat	Higher-risk chronic myelomonocytic leukemia (HR-CMML), low-blast acute myeloid leukemia (LB-AML) and higher-		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
Polihexanide	For the treatment of acanthamoeba keratitis	x	jun.22
Quizartinib	Treatment for acute myeloid leukaemia	x	nov.18
Repotrectinib	ROS1+ Advanced Non-Small Cell Lung Cancer (NSCLC) or NTRK+ Advanced Solid Tumors		nov.21
Retifanlimab	Treatment of locally advanced or metastatic squamous carcinoma of the anal canal (SCAC) who have progressed	x	mar.21
Romidepsin	Treatment of peripheral T-cell lymphoma (PTCL) in adult patients who have received at least one prior therapy.		feb.22
Rozanolixizumab	Treatment of generalised myasthenia gravis (gMG)	x	des.22
Rovalpituzumab tesirine	Small cell lung cancer (SCLC)		jan.18

Ruxolitinib	Treatment of non-segmental vitiligo - Kun denne indikasjonen		okt.21
Siponimod	Indicated for the treatment of thrombocytopenia - Kun denne indikasjonen		okt.18
Sirolimus	Treatment of angiofibroma associated with tuberous sclerosis complex - Kun denne indikasjonen	x	feb.22
Sodium phenylbutyrate / ursodoxicoltaurine	Treatment of amyotrophic lateral sclerosis (ALS)	x	apr.22
Spesolimab	Treatment of flares in adult patients with generalised pustular psoriasis		okt.21
Surufatinib	Treatment of progressive neuroendocrine tumours		jul.21
Talazoparib	Treatment of prostatecancer - Kun denne indikasjonen		mar.18
Talquetamab	Treatment of multiple myeloma	x	sep.22
Tazemetostat	Treatment of malignant mesothelioma and Epithelioid sarcoma		mai.21
Tofersen	Amyotrophic lateral sclerosis (ALS), treatment of adults with amyotrophic lateral sclerosis (ALS), associated with a		nov.21, des.22
Toripalimab	Combination treatment for metastatic or recurrent locally advanced nasopharyngeal carcinoma and for		des.22
Trametinib	Treatment of paediatric patients aged 1 year and older with glioma - Kun denne indikasjonen	x	okt.22
Trastuzumab duocarmazine	Treatment of HER2 (Human Epidermal Growth Factor Receptor 2)-positive metastatic breast		aug.22
Tremelimumab	For use in combination with durvalumab for the treatment of adults with unresectable hepatocellular carcinoma. Treatment of adults with metastatic NSCLC with no sensitising epidermal growth factor receptor (EGFR) mutation or anaplastic lymphoma kinase (ALK) genomic tumour aberrations		mar.22/ feb.22
Ublituximab	Treatment of relapsing forms of multiple sclerosis (RMS)		feb.22
Valoctocogene roxaparvovec	Treatment of haemophilia A	x	feb.20 og ju.21
Valemetostat	Ved tilbakevendende/refraktær (R/R) adult T-cell leukemia/lymphoma (ATLL).		apr.22

Vamorolone	Treatment of Duchenne muscular dystrophy (DMD)	x	nov.22
Veliparib	Treatment of BRCA 1, BRCA 2, BARD1 and/or PALB2 mutated cancer		mar.18
Zilucoplan	treatment of generalised myasthenia gravis in adults	x	okt.22