

## Nye legemidler som ikke har markedsføringstillatelse

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

Oppdatert: 03.07.2025

Virkestoff	Indikasjon per nå. (Samt mulig andre indikasjoner i fremtiden.)	Orphan medicinal product (Se informasjon om COMP (Komiteen for legemidler mot sjeldne sykdommer) på <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
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
allogeneic faecal microbiota, pooled	Treatment of patients with acute-graft-versus-host disease (aGvHD)		jul.25
alpelisib	Treatment of adult and paediatric patients aged 2 years and older with severe or life-threatening manifestations of PIK3CA-related overgrowth spectrum (PROS)./ Treatment of patients with severe manifestations of PIK3CArelated overgrowth spectrum - <b>Kun denne indikasjonen</b>	x	jun.25/ 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
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
autologous melanoma-derived tumor infiltrating lymphocytes, ex vivo-expanded	Treatment of melanoma		mai.25
bardoxolone methyl	Treatment of chronic kidney disease (CKD)	x	okt.21 /des.21
belantamab mafodine	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
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
brensocatib	Treatment of non-cystic fibrosis bronchiectasis		mai.25
camizestrant	treatment of locally advanced or metastatic breast cancer		jul.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
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
diazoxide choline	Treatment of adult and paediatric patients with Prader-Willi syndrome	x	jun.25
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
dorocubicel / allogeneic umbilical cord-derived CD34- cells, non-expanded	Treatment of adult patients with haematological malignancies	x	jul.24
doxycitine / 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) - <b>Kun denne indikasjonen</b>	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 - <b>Kun denne indikasjonen</b>	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
glepaglutide	Treatment of Short Bowel Syndrome		jul.25
glutamine	Treatment of sickle cell disease		mar.18
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 - <b>Kun denne indikasjonen</b>	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
lenadogene nolpharvovec	Treatment of vision loss due to Leber Hereditary Optic Neuropathy (LHON)	x	nov.20
lenacapavir	Pre-exposure prophylaxis to prevent HIV-1 ( <b>kun denne indikasjonen</b> )		mar.25
leniolisib	Treatment of activated phosphoinositide 3-kinase delta syndrome (APDS)	x	nov.22
lifileucel	Treatment of unresectable or metastatic melanoma		sep.24
linerixibat	Treatment of cholestatic pruritus with primary biliary cholangitis		jul.25
lurbinectedin	Treatment patients with extensive-stage small cell lung cancer (ES-SCLC)		jul.25

LUT014	Topical BRAF Inhibitor Therapy for EGFR Inhibitor–Induced Acneiform Rash		nov.23
mavorixafor	Treatment of WHIM syndrome	x	jan.25
mirdametinib	Treatment of neurofibromatosis type 1		sep.24
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
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
naporafenib	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
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). <b>Kun denne indikasjonen</b>		aug.20
obnitix	Treatment of RR-aGVHD		apr.24
olezarsen	Treatment of familial chylomicronemia		sep.24
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) - <b>Kun denne indikasjonen</b>	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 dysfunctionassociated steatohepatitis (MASH) with liver fibrosis		apr.24
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 <b>(kun denne indikasjonen)</b>	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
sasanlimab	Indicated for treatment of bladder cancer		jun.25
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
siponimod	Indicated for the treatment of thrombocytopenia - <b>Kun denne indikasjonen</b>		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
tolebrutinib	Treatment of non-relapsing secondary progressive multiple sclerosis (nrSPMS) in adults		mai.25
tovorafenib	Treatment of paediatric low grade glioma		mar.25
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/or 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