

## Nye legemidler som ikke har markedsføringstillatelse

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

Oppdatert: 13.06.2019

Virkestoff	Indikasjon per nå. (Samt mulig andre indikasjoner i fremtiden.)	Orphan medicinal product (* Se informasjon nederst)	Oppført på listen
Isatuximab	Treatment of patients with multiple myeloma (MM)	x	jun.19
Deferiprone	Treatment of neurodegeneration with brain iron accumulation ( <b>Kun denne indikasjonen</b> )	x	jun.19
Givosiran	Indicated for the treatment of acute hepatic porphyria in adults and adolescents		jun.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
Glasdegib	Treatment of newly diagnosed de novo or secondary acute myeloid leukaemia	x	jun.19
Treprostinil sodium	Treatment of thromboembolic pulmonary hypertension (CTEPH) (kun denne spesifikke indikasjonen)	x	mar.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 indikasjonen)		mar.19
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

Darolutamide	Non-metastatic castration resistant prostate cancer		mar.19
Gliteritinib	Treatment of patients who have relapsed or refractory acute myeloid leukemia (AML) with a FLT3 mutation	x	mar.19
Imlifidase	Indicated for desensitization treatment of highly sensitized adult kidney transplant patients with positive crossmatch against an available deceased donor	x	mar.19
Fenfluramine	Treatment of seizures associated with Dravet syndrome in children aged 2 years to 17 years and adults.	x	mar.19
Brolucizumab	Treatment of neovascular (wet) age-related macular degeneration (AMD)		mar.19
Upadacitinib	Treatment of moderate to severe active rheumatoid arthritis		feb.19
Gilteritinib	Treatment in adults of FMS-like tyrosine kinase 3 (FLT3) mutation positive patients with relapsed or refractory acute myeloid leukemia (AML).	x	feb.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
Tagraxofusp	Treatment of adult patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN)	x	feb.19
Diclofenamide	Treatment of periodic paralysis		feb.19
Selinexor	Treatment of patients with relapsed refractory multiple myeloma (RRMM)	x	jan.19
Polatuzumab vedotin	Treatment of mature B cell lymphomas		jan.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

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
Osilodrostat	Treatment of Cushing's syndrome	x	jan.19
Imipenem/ Cilastatin/ Relebactam	Treatment of bacterial infections due to gram-negative microorganisms		jan.19
Onasemnogene abeparvovec	Treatment of spinal muscular atrophy (SMA)	x	nov.18
Quizartinib	Treatment for acute myeloid leukaemia	x	nov.18
Plazomicin	Treatment of Complicated urinary tract infection (cUTI), including pyelonephritis; treatment of Bloodstream infection (BSI); treatment of infections due to Enterobacteriaceae		nov.18
Depatuximab mafodotin	Treatment of glioblastoma (GBM)		nov.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
Omadacycline tosylate	Treatment of community-acquired bacterial pneumonia (CABP) and acute bacterial skin and skin structure		okt.18
Siponimod	Treatment of secondary progressive multiple sclerosis (SPMS)		okt.18
autologous cd34+ cell enriched population that contains hematopoietic stem cells transduced with lentiglobin bb305 lentiviral vector encoding the beta-a-t87q-globin gene	Treatment of transfusion-dependent $\beta$ -thalassaemia (TDT)	x	okt.18
Siponimod	Indicated for the treatment of thrombocytopenia		okt.18
Dolutegravir / lamivudine	Treatment of Human Immunodeficiency Virus type 1 (HIV-1)		okt.18

Adeno-associated viral vector serotype 9 containing the human SMN gene (AVXS-101)	Treatment of paediatric patients diagnosed with spinal muscular atrophy Type 1		okt.18
Olipudase alfa	Treatment of non-neurological manifestations of acid sphingomyelinase deficiency		okt.18
Larotrectinib	Treatment of adult and paediatric patients with locally advanced or metastatic solid tumours	x	sep.18
Ibalizumab	Treatment of adults infected with HIV-1 resistant to at least 1 agent in 3 different classes		sep.18
Ravulizumab	Treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH)	x	aug.18
L-lysine hydrochloride / l-arginine hydrochloride	Reduction of renal radiation exposure during Peptide-Receptor Radionuclide Therapy (PRRT) with lutetium (177Lu) oxodotreotide		jul.18
Enasidenib	Treatment of acute myeloid leukaemia (AML)	x	jul.18
Angiotensin ii	Treatment of hypotension in adults with distributive or vasodilatory shock who remain hypotensive despite fluid and vasopressor therapy		jul.18
Delafloxacin	Treatment of Acute Bacterial Skin and Skin Structure Infection (ABSSSI) in adults		jun.18
Cemiplimab	Monotherapy, indicated for the treatment of patients with metastatic cutaneous squamous cell carcinoma		mai.18
Turoctocog alfa pegol	Treatment and prophylaxis of bleeding in patients with haemophilia A	x	mai.18
Glutamine	Treatment of sickle cell disease		mar.18
Trientine dihydrochloride	Treatment of Wilson's disease	x	mar.18
Selumetinib	Neurofibromatosis		mar.18
Avapritinib	Treatment of mastocytosis and GIST		mar.18
Ipatasertib	Treatment of breastcancer		mar.18
Veliparib	Treatment of BRCA 1, BRCA 2, BARD1 and/or PALB2 mutated cancer		mar.18
Talazoparib	Treatment of breastcancer and prostatecancer		mar.18
Copanlisib	Treatment of non-hodgkin lymphoma		mar.18
Enasidenib	Treatment of acute myeloid leukaemia		mar.18
Acalabrutinib	Treatment of mantel cell lymphoma		mar.18

Treosulfan	Conditioning treatment prior to allogeneic haematopoietic stem cell transplantation (alloHSCT)	x	mar.18
Cannabidiol	Adjunctive therapy of seizures associated with Lennox-Gastaut syndrome (LGS) or Dravet syndrome (DS),	x	mar.18
Avatrombopag Maleat	Avatrombopag is indicated for the treatment of thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo a procedure.		mar.18
Rovalpituzumab tesirine	Small cell lung cancer (SCLC)		jan.18
Edaravone	Amyotrophic lateral sclerosis (ALS)		jan.18
Romosozumab	Treatment of osteoporosis		jan.18
Entolimod	Treatment of acute radiation syndrome	x	nov.17
Botulinum toxin type a	Temporary improvement in the appearance of moderate to severe vertical lines between the eyebrows		jul.17
Autologous T cells transduced with retroviral vector encoding an anti-CD19 CD28/CD3-zeta chimeric antigen receptor	Intended for the treatment of adult patients with diffuse large B-cell lymphoma (DLBCL) who have not responded to their prior therapy, or have had disease progression after autologous stem cell transplant (ASCT)	x	jun.17
* Se informasjon om COMP (komiteen for legemidler mot sjeldne sykdommer) på <a href="http://www.legemiddelverket.no">www.legemiddelverket.no</a>			