

Nye legemidler som ikke har markedsføringstillatelse

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

Oppdatert: 13.11.2018

Virkestoff	Indikasjon per nå. (Samt mulig andre indikasjoner i fremtiden.)	Orphan medicinal product (* Se informasjon nederst)	Oppført på listen
Depatuximab mafodotin	Treatment of glioblastoma (GBM)		nov.18
Alpelisib	Breast cancer; advanced hormone receptor positive (HR+), HER2-negative in men and postmenopausal women - second-line with fulvestrant		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 β -thalassaemia (TDT)	x	okt.18
Fostamatinib	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
Risankizumab	Treatment of psoriasis in adults		jun.18
Crisaborole	Treatment of mild to moderate atopic dermatitis		jun.18
Delafloxacin	Treatment of Acute Bacterial Skin and Skin Structure Infection (ABSSSI) in adults		jun.18
Axalimogene filolisbac	Treatment of cervical cancer		mai.18
Pegvaliase	Treatment of adults with phenylketonuria (PKU) who have inadequate blood phenylalanine control	x	mai.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
Dacomitinib	First-line treatment of adults with locally advanced or metastatic non-small cell lung cancer (NSCLC) with epidermal growth factor receptor (EGFR)-activating mutations.,		mar.18
Trientine dihydrochloride	Treatment of Wilson's disease	x	mar.18
Selumetinib	Neurofibromatosis		mar.18
Masitinib	Treatment of amyotrophic lateral sclerosis (ALS). Se fotnote under**		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
Canakinumab	Prevention of major cardiovascular events		mar.18
Lusutrombopag	Treatment of thrombocytopenia		mar.18
Lorlatinib	Treatment of adult patients with anaplastic lymphoma kinase (ALK)-positive advanced non-small cell lung cancer (NSCLC)		mar.18
Asparaginase	Treatment of acute lymphoblastic leukaemia	x	mar.18
Cannabidiol	Adjunctive therapy of seizures associated with Lennox-Gastaut syndrome (LGS) or Dravet syndrome (DS),	x	mar.18
Fremanezumab	Prevention of episodic and chronic migraine		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
Lanadelumab	Routine prevention of angioedema attacks and the control of symptoms of hereditary angioedema (HAE) in patients aged 12 years and older	x	feb.18
Regn2810	Metastatic Cutaneous Squamous Cell Carcinoma (CSCC) indicated for the treatment of patients with metastatic cutaneous squamous cell carcinoma (mCSCC), or with locally advanced cutaneous squamous cell carcinoma (laCSCC) who are not candidates for surgery		feb.18
Rovalpituzumab tesirine	Small cell lung cancer (SCLC)		jan.18
Edaravone	Amyotrophic lateral sclerosis (ALS)		jan.18
Avacopan	Induction of response in adult patients with granulomatosis with polyangiitis (Wegener's) (GPA) or microscopic polyangiitis (MPA)	x	jan.18
Romosozumab	Treatment of osteoporosis		jan.18
Apalutamide	Intended for the treatment of adult men with NM-CRPC who are at high risk of developing metastatic disease.		jan.18

Doravirine	Treatment of adults infected with HIV-1 without past or present evidence of viral resistance to treatment of adults infected with HIV-1 without past or present evidence of viral resistance to doravirine,		des.17
Doravirine / lamivudine / tenofovir disoproxil	Treatment of adults infected with HIV-1 without past or present evidence of viral resistance to doravirine, lamivudine, or tenofovir		des.17
Galcanezumab	Prophylaxis of migraine		des.17
Macimorelin	Diagnosis of Adult growth hormone deficiency (AGHD)		des.17
Entolimod	Treatment of acute radiation syndrome	x	nov.17
Mogamulizumab	Treatment of cutaneous T-cell lymphoma	x	nov.17
Damoctocog alfa pegol	Treatment and prophylaxis of haemophilia A	x	okt.17
Pacritinib	Treatment of disease-related splenomegaly and control of symptoms in patients with primary myelofibrosis (PMF), post-polycythemia vera myelofibrosis (PPV-MF), or post-essential thrombocythemia myelofibrosis (PET-MF) who have thrombocytopenia (platelet counts $\leq 100,000 /\mu\text{L}$)		jul.17
Botulinum toxin type a	Temporary improvement in the appearance of moderate to severe vertical lines between the eyebrows		jul.17
Meropenem / vaborbactam	Treatment of infections		jul.17
Ivacaftor/ tezacaftor	The treatment of patients with CF aged 12 years and older who have at least one F508del mutation in the CFTR gene, and a second mutation that is responsive to tezacaftor/ivacaftor		jun.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 www.legemiddelverket.no			

****Footnote/ Masitinib:**

http://www.ema.europa.eu/docs/en_GB/document_library/Summary_of_opinion_-_Initial_authorisation/human/04398/WC500247742.pdf