Nye legemidler som ikke har markedsføringstillatelse

For legemidler oppført på listen gjelder retningslinjens vilkår <u>uavhengig av indikasjon for bruken</u>. Oppdatert: 04.04.2024

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.legemi ddelverket.n o	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
acoramidis	Treatment of wild-type or variant transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR- CM)	х	mar.24
allogeneic bone marrow derived mesenchymal stromal cells, ex- vivo expanded	Treatment of graft-versus-host disease.	x	sep.23
alpelisib	Treatment of patients with severe manifestations of PIK3CArelated overgrowth spectrum - Kun denne indikasjonen	x	aug.22
apadamtase alfa	Treatment of congenital thrombotic thrombocytopenic purpura (cTTP) due to ADAMTS13 deficiency	x	jun.23
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	Repair of cartilage defects of the knee		
chondrocytes in vitro expanded	joint		
			sep.20
autologous cartilage-derived	Repair of symptomatic, localised, full-		
articular	thickness cartilage defects of the knee		
chondrocytes, in-vitro expanded	joint grade III or IV		
			jan.24
autologous glioma tumor cells,	Treatment of glioma		
inactivated / autologous glioma tumor cell lysates, inactivated /			
allogeneic glioma tumor cells,			
inactivated / allogeneic glioma		x	
tumor cell lysates, inactivated		ň	
			okt.20
bardoxolone methyl	Treatment of chronic kidney disease		
	(CKD)	x	okt.21
			/des.21
belinostat	Relapserad/refraktärt T-cellslymfom		24
			mar.24
belumosudil	versus host disease (GvHD)		nov.22
beremagene-geperpavec	Treatment of patients from birth with	x	des.23
	dystrophic		/jun.23
belzutifan	Treatment of adult patients with		
	advanced renal		jan.24
	Treatment of neovascular (wet)		
bevacizumab	age related macular degeneration		
	(nAMD) - Kun denne indikasjonen		feb.23
capivasertib			
	Indicated in combination with		
	fulvestrant for the treatment of adult		
	patients with hormone receptor (HR)		
	positive, human epidermal growth		
	factor receptor 2 (HER2) negative		
	(defined as IHC 0 or 1+, or IHC 2+/ISH-) locally advanced or metastatic breast		
	cancer following recurrence or		
	progression on or after an endocrine		
	based regimen		jun.23
			jun.25

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 ≥ 12		
	years of age haemophilia B (congenital		
	factor IX deficiency) with FIX inhibitors		
	of any age.		feb.23
copanlisib			100.20
	Treatment of non-hodgkin lymphoma		mar.18
	Treatment of adult patients with	X	
copanlisib	marginal zone lymphoma	х	jul.22
	Treatment of paroxysmal nocturnal		
crovalimab	haemoglobinuria		aug.23
danicopan	Treatment of extravascular haemolysis		
	(EVH) in patients with paroxysmal	х	
	nocturnal haemoglobinuria		apr.23
daprodustat			·
•	Treatment of anemia assiciated 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	х	
	indikasjonen		jun.19
delandistrogene moxeparvovec	Treatment of Duchenne muscular		-
0	dystrophy		aug.23
depatuximab mafodotin	Treatment of glioblastoma (GBM)		nov.18
diclofenamide	Treatment of periodic paralysis		feb.19
Diflunisal	Treatment of ATTR amyloidosis	Х	mar.24
duvelisib	Treatment of adult patients with small		
	lymphocytic lymphoma (SLL) - Kun	х	
	denne indikasjonen		feb.20
edaravone	Amyotrophic lateral sclerosis (ALS)		jan.18
efanesoctocog alfa	Treatment and prophylaxis of bleeding		
	in patients with haemophilia A		jun.23
eflornithine / sulindac	Treatment of familial adenomatous		-
	polyposis	х	jul.20
	Treatment of primary biliary cholangitis		
elafibranor	(PBC)	х	nov.23
enasidenib			
	Treatment of acute myeloid leukaemia		mar.18
entolimod			
	Treatment of acute radiation syndrome		nov.17

eplontersen	Treatment of adult patients with		1 1
	polyneuropathy associated with	X	
	hereditary transthyretin-mediated	Х	
	amyloidosis (ATTRv)		nov.23
erdafitinib	Treatment of adults with locally		
	advanced or metastatic urothelial		nov.19, nov.23
	carcinoma		
ferumoxytol	Intravenous treatment of iron deficiency		
	anaemia (IDA)		jun.22
fidanacogene elaparvovec			
	Indicated for the treatment of severe	х	
	and moderately severe haemophilia B		jun.23
fruquintinib	Treatment of metastatic colorectal		
	cancer		jul.23
garadacimab	Routine prevention of attacks of	х	
	hereditary angioedema (HAE)	^	des.23
glutamine	Treatment of sickle cell disease		mar.18
govorestat	Treatment of adults and		
	children aged 2 years and older with a		
	confirmed diagnosis of classic	х	
	galactosemia		jan.24
idebenone	Treatment of respiratory dysfunction in		
	patients with Duchenne muscular		
	dystrophy (DMD) not using		
	glucocorticoids - Kun denne	х	
	indikasjonen		
			aug.19
imetelstat	Treatment of adult patients with		
	transfusion-dependent anaemia due to		
	very low, low and intermediate-risk		
	myelodysplastic syndromes (MDS) with	х	
	ring sideroblasts		
			nov.23
infigratinib	Treatment of cholangiocarcinoma		
		Х	des.21
ipatasertib	Treatment of breastcancer		mar.18
iptacopan	Treatment of Paroxysmal Nocturnal		
	Haemoglobinuria (PNH)		mai.23
lazertinib	Treatment of adult patients with		
	advanced nonsmall cell lung cancer		
	(NSCLC)		mar.24
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	x	
	Hereditary Optic Neuropathy (LHON)		nov.20
leniolisib	Treatment of activated		
	phosphoinositide 3-kinase delta	x	
	syndrome (APDS)		nov.22
linvoseltamab	Monotherapy for the treatment of adult		
	patients with relapsed or refractory		
	multiple myeloma		mar.24
LUT014			
	Topical BRAF Inhibitor Therapy for EGFR		
	Inhibitor–Induced Acneiform Rash		nov.23
macitentan / Tadalafil (kun			
denne spesifikk kombinasjon)	treatment of pulmonary arterial		
	hypertension (PAH) in adult patients		aug.23
marstacimab	Indicated for routine prophylaxis of		
	bleeding episodes in patients with	x	
	haemophilia A or haemophilia B		nov.23
mirvetuximab soravtansine	Treatment of ovarian, fallopian tube, or	x	
	primary peritoneal cancer	^	nov.23
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
naporafenib	Treatment of advanced solid tumours		
	including ovarian cancer		okt.21
nemolizumab	Treatment of moderate-to-severe atopic		
	dermatitis and for the treatment of		
	prurigo nodularis		mar.24
nirogacestat	Treatment of desmoid tumours	х	apr.24
odevixibat	Treatment of cholestatic pruritus in		
	Alagille syndrome (ALGS) Kun denne		
	indikasjonen		jan.24
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

omecamtiv mecarbil	1		
	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		
	Treatment of adult patients with		apr.21
parsaclisib	relapsed or refractory marginal zone		
	lymphoma (MZL)	x	fab 22
	Treatment of geographic atrophy (GA)		feb.22
pegcetacoplan	secondary to age-related macular		
	degeneration (AMD) - Kun denne	x	
	indikasjonen		feb.23
novenedistat	indikasjonen		160.23
pevonedistat	Higher-risk chronic myelomonocytic		
	leukemia (HR-CMML), low-blast acute		
	myeloid leukemia (LB-AML) and higher		
	risk myelodysplastic syndromes		200
nlazamiain			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	x	
	resection is potentially associated with		
	worsening functional limitation or		
	severe morbidity		
	severe morbially		mar.19
plazomicin	Treatment of Complicated urinary tract		11111.15
piazonnem	infection (cUTI), including		
	pyelonephritis; treatment of		
	Bloodstream infection (BSI);		
	treatment of infections due to		
	Enterobacteriaceae		nov.18
polihexanide	For the treatment of acanthamoeba		1101.10
pointexanide	keratitis	x	jun.22
repotrectinib			Jun.22
	Treatment of ROS1-positive locally		
	advanced or		
	metastatic non-small cell lung cancer		
	(NSCLC)		
	and for solid tumours; ROS1+ Advanced		
	Non-Small Cell Lung Cancer (NSCLC) or		jan.24,
	NTRK+ Advanced Solid Tumors		nov.21
			100.21

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	х	
	anal canal (SCAC) who have progressed		
	on or who are intolerant of platinum		mar.21
retifanlimab	Treatment of Merkel cell carcinoma		
	(MCC).	х	apr.23
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
savolitinib	monotherapy for the treatment of adult		
	patients with relapsed or refractory		
	multiple myeloma		mar.23
Seladelparlysindihydrat			
	A peroxisome proliferator receptor delta		
	(PPAR δ) activator indicated for the		
	treatment of primary biliary cholangitis		
	(PBC) including pruritus in adults		
	without cirrhosis or with compensated		
	cirrhosis (Child-Pugh A) in combination		
	with ursodeoxycholic acid (UDCA) who		
	have an inadequate response to UDCA		
	alone, or as monotherapy in those		
	unable to tolerate UDCA.		apr.24
serplulimab	First-line treatment of adult patients		
•	with extensive-stage small cell lung	х	
	cancer (ES-SCLC)		apr.23
sipavibart			· ·
	Pre-eksponeringsprofylakse mot covid-		
	19-sykdom hos spesielt sårbare grupper		
	hvor vaksinasjon ikke gir tilstrekkelig		
	beskyttelse mot alvorlig sykdom.		mar.24
siponimod	Indicated for the treatment of		
	thrombocytopenia - Kun denne		
	indikasjonen		okt.18
sodium phenvlbutvrate/			okt.18
sodium phenylbutyrate/ ursodoxicoltaurine		x	okt.18

sotatercept	Treatment of pulmonary arterial	x	
	hypertension in adults	~	sep.23
sugemalimab	Treatment of non-small cell lung cancer (NSCLC)		mar.23
surufatinib	Treatment of progressive		
	neuroendocrine tumours		jul.21
tazemetostat	Treatment of malignant mesothelioma		
	and Epithelioid sarcoma		mai.21
tiratricol	Treatment of monocarboxylate		
	transporter 8 (MCT8) deficiency	х	nov.23
tisotumab vedotin	treatment of adult patients with recurrent or metastatic cervical cancer with disease progression on or after systemic therapy		mar.24
tofersen	A must mark in lateral calcussis (ALC)		
	Amyotrophic lateral sclerosis (ALS),		
	Treatment of adults with amyotrophic		
	lateral sclerosis (ALS), associated with a		nov.21, des.22
	mutation in the superoxide dismutase 1		des.zz
toripalimab	Combination treatment for metastatic or recurrent locally advanced nasopharyngeal carcinoma and for		
	metastatic or recurrent oesophageal		des.22
trastuzumab duocarmazine	Treatment of HER2 (Human Epidermal		uc3.22
	Growth Factor Receptor 2)-positive metastatic breast		aug.22
troriluzole			
	Treatment of adult patients with	x	
	spinocerebellar ataxia genotype 3 (SCA3		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/orr PALB2 mutated cancer		mar.18
zolbetuximab			
	Treatment of locally advanced		
	unresectable or metastatic HER2	х	
	negative gastric or gastro-oesophageal		
	junction (GEJ) adenocarcinoma		aug.23