NYE METODER

Anmodning om vurdering av legemiddel i Nye metoder

Skjema for leverandører

En leverandør som ønsker offentlig finansiering av et legemiddel/legemiddelindikasjon i den norske spesialisthelsetjenesten, skal anmode om vurdering i Nye metoder ved å fylle ut dette skjemaet.

Utfylt anmodningsskjema sendes til Nye metoder: nyemetoder@helse-sorost.no

Leverandøren skal på anmodningstidspunktet både ha et forslag til type helseøkonomisk analyse og en plan for når de leverer dokumentasjonen. Merk at dokumentasjon i henhold til oppdraget fra Bestillerforum for nye metoder må leveres inn senest 12 måneder etter anmodningstidspunktet.

Hele anmodningsskjemaet skal fylles ut. Mer informasjon og veiledning finnes i artikkelen For leverandører (nyemetoder.no)

Merk: Skjemaet vil bli publisert i sin helhet på nyemetoder.no.

Innsender er klar over at skjemaet vil bli publisert i sin helhet (må krysses av): ☒

Fyll ut dato for innsending av skjema: 24.07.2026

1 Kontaktopplysninger	
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2 Legemiddelinformasjon og indikasjon	
2.1 Hva gjelder anmodningen? *Kryss av for hva anmodningen gjelder*	Et nytt virkestoff ☒ En indikasjonsutvidelse / ny indikasjon ☐ En ny styrke eller formulering ☐
2.2 Hvilken indikasjon gjelder anmodningen?	Norwegian: Xolremdi (mavorixafor) er indisert hos pasienter fra 12 år og oppover for behandling av WHIM-syndrom (vorter,

Indikasjonen skal oppgis på norsk. Hvis prosess for godkjenning pågår, oppgi også indikasjon på engelsk. *Merk: Leverandør skal anmode om vurdering av hele indikasjonen som de har fått godkjent eller søker om godkjenning for. Dersom leverandør foreslår en avgrensning til undergrupper, må dette begrunnes og leverandør må levere dokumentasjonen som trengs for å foreta en vurdering av undergruppen i tillegg til dokumentasjonen for hele indikasjonen.*	hypogammaglobulinemi, infeksjoner og myelokatheksis) for å øke antallet sirkulerende modne nøytrofiler og lymfocytter. English: Xolremdi (mavorixafor) is indicated in patients 12 years of age and older for the treatment of WHIM syndrome (warts, hypogammaglobulinemia, infections and myelokathexis) to increase the number of circulating mature neutrophils and lymphocytes (1).
2.3 Handelsnavn	Xolremdi
2.4 Generisk navn/virkestoff	Mavorixafor
2.5 ATC-kode	L03AX24 (1)
2.6 Administrasjonsform og styrke *Oppgi også forventet dosering og behandlingslengde* *Skriv kort*	Oral use. The dosing differs for patients whose weight below 50kg. The recommended dose according to SmPC is: - Weight more than 50 kg: 400 mg (four 100 mg capsules) orally once daily on an empty stomach after an overnight fast, and at least 30 minutes before food. - Weight less than or equal to 50 kg: - 300 mg (three 100 mg capsules) orally once daily on an empty stomach after an overnight fast, and at least 30 minutes before food based on SmPC (1) - 200 mg once daily based on 4WHIM trial (2) Pharmacokinetic data from the supportive open-label Phase 2 trial, shows that mean ANC levels for doses 50 to 200 mg generally remained below the clinical benefit threshold of 500 cells/μL during the 24-hour dosing interval. For 300 mg and 400 mg, mean ANC levels rose above the threshold by approximately 1hour post-dose and remained above or at the threshold over the entire dosing interval. A mavorixafor dose of 300/400 mg QD was required to achieve $AUC_{ANC} \geq 600$/μL and $AUC_{ALC} \geq 1\ 000$/μL (1).
2.7 Farmakoterapeutisk gruppe og virkningsmekanisme. *Skriv kort*	Pharmacotherapeutic group: Immunostimulants, Other immunostimulants (1) Mechanism of action: Mavorixafor is a CXC Chemokine Receptor 4 (CXCR4) antagonist that blocks the binding of the CXCR4 ligand, stromal-derived factor-1α (SDF-1α)/CXC Chemokine Ligand 12 (CXCL12). SDF-1/CXCR4 plays a role in trafficking and homing of leukocytes to and from the bone

	marrow compartment. Gain of function mutations in the CXCR4 receptor gene that occur in patients with WHIM syndrome lead to increased responsiveness to CXCL12 and retention of leukocytes in the bone marrow. Mavorixafor inhibits the response to CXCL12 in both wild-type and mutated CXCR4 variants associated with WHIM syndrome. Treatment with mavorixafor results in increased mobilisation of neutrophils and lymphocytes and monocytes from the bone marrow into peripheral circulation.

3 Historikk – virkestoff og indikasjon

3.1 Har Nye metoder behandlet metoder med det aktuelle virkestoffet tidligere? *Hvis ja, oppgi ID-nummer til metoden/metodene i Nye metoder*	Ja ☐ Nei ☒ ID-nummer: Klikk eller trykk her for å skrive inn tekst.
3.2 Er du kjent med om andre legemidler/virkestoff er vurdert i Nye metoder til samme indikasjon? *Hvis ja, oppgi ID-nummer til metoden/metodene i Nye metoder*	Ja ☐ Nei ☒ ID-nummer: Klikk eller trykk her for å skrive inn tekst.
3.3 Er du kjent med om det er gjennomført en metodevurdering i et annet land som kan være relevant i norsk sammenheng? *Hvis ja, oppgi referanse*	Ja ☐ Nei ☒ Referanse: Klikk eller trykk her for å skrive inn tekst.

4 Status for markedsføringstillatelse (MT) og markedsføring

4.1 Har legemiddelet MT i Norge for en eller flere indikasjoner? *Hvis ja - skriv inn dato for norsk MT for den første indikasjonen*	Ja ☒ Nei ☐ Dato for MT for første indikasjon: 27.04.2026
4.2 Markedsføres legemiddelet i Norge?	Ja ☐ Nei ☒
4.3 Har legemiddelet MT i Norge for anmodet indikasjon? *For alle metoder: Fyll ut prosedyrenummer i EMA (det europeiske legemiddelbyrået)*	MT i Norge: Ja ☒ Nei ☐ Prosedyrenummer i EMA: EMA procedure number: EMEA/H/C/006496 27 April 2026

Hvis metoden ikke har MT i Norge, fyll ut forventet tidspunkt (måned/år) for CHMP opinion i EMA. *Hvis metoden har MT i Norge, fyll ut dato for MT*	**Hvis metoden ikke har MT**: Forventet tidspunkt for CHMP opinion i EMA (måned/år): Klikk eller trykk her for å skrive inn tekst. Forventet tidspunkt for markedsføringstillatelse (MT) for den aktuelle indikasjonen i Norge (måned/år): Klikk eller trykk her for å skrive inn tekst.
	Hvis metoden har MT: Dato for MT i Norge for den aktuelle indikasjonen: Klikk eller trykk for å skrive inn en dato.
4.4 Har legemiddelet en betinget markedsføringstillatelse for anmodet indikasjon? *Hvis ja, fyll ut en beskrivelse av hva som skal leveres til EMA og når.*	Ja ☐ Nei ☒ Beskrivelse: Klikk eller trykk her for å skrive inn tekst.
4.5 Har anmodet indikasjon vært i «accelerated assessment» hos EMA?	Ja ☐ Nei ☒
4.6 Har legemiddelet «orphan drug designation» i EMA? *Hvis ja, fyll ut dato*	Ja ☒ Nei ☐ Dato for «orphan drug designation»: 25.07.2019

5 Ordning for forenklet vurdering av PD-(L)1-legemidler

5.1 Er legemiddelet registrert i Nye metoders ordning «Forenklet vurdering av PD-(L)1-legemidler"?	Ja ☐ Nei ☒

6 Sammenlignbarhet og anbud

6.1 Finnes det andre legemidler med lignende virkningsmekanisme og /eller tilsvarende effekt til den aktuelle indikasjonen?	Ja ☒ Nei ☐ Kommentar: Similar to mavorixafor, plerixafor is also a CXCR4 antagonist, which is used as an off-label treatment for the treatment of WHIM syndrome, as confirmed by a Nordic clinical expert (3). Plerixafor is available in Norway for

	haematopoietic stem cell mobilization in lymphoma and multiple myeloma, which is used as a hospital-administered treatment (subcutaneous injection) within autologous transplant programs (4). It has not been assessed by Nye Metoder.
6.2 Vurderer leverandør at legemiddelet i anmodningen er sammenlignbart med et eller flere andre legemidler som Nye metoder har besluttet å innføre til den samme indikasjonen? *Hvis ja, hvilke(t)? Oppgi ID-nummer på metoden/metodene i Nye metoder*	Ja ☐ Nei ☒ Legemiddel og ID-nummer: Klikk eller trykk her for å skrive inn tekst.
6.3 Er det eksisterende anbud på terapiområdet som kan være aktuelt for legemiddelet?	Ja ☐ Nei ☒ Kommentar: Klikk eller trykk her for å skrive inn tekst.

7 Nordisk samarbeid JNHB (Joint Nordic HTA-bodies)

7.1 Er anmodet indikasjon aktuell for utredning i det nordiske HTA-samarbeidet JNHB? *Hvis nei, begrunn kort*	Ja ☐ Nei ☒ Begrunnelse: Mavorixafor is not considered to be a candidate for JNHB, as it is administered in the outpatient setting in Sweden and Finland. According to JNHB, products used in inpatient care are generally considered more suitable for joint assessment (5). Furthermore, a standard reimbursement application to HILA would still be required in Finland for an outpatient product as JNHB cannot replace the process.

8 Europeisk samarbeid om vurdering av relativ effekt og sikkerhet (HTAR)

8.1 Er anmodet legemiddel/indikasjon omfattet av regelverket for utredning av relativ effekt og sikkerhet i europeisk prosess (HTAR)? *Hvis ja, fyll ut dato for søknad om MT til EMA*	Ja ☐ Nei ☒ Dato for søknad til EMA: Klikk eller trykk for å skrive inn en dato.

9 Helseøkonomisk dokumentasjon og forslag til helseøkonomisk analyse

9.1 Hvilken type helseøkonomisk analyse foreslår leverandøren?	Cost utility analysis is proposed for this submission.

F.eks. kostnad-per-QALY analyse eller kostnadsminimeringsanalyse. *Begrunn forslaget*	This is because cost utility analysis reflects both the survival and health-related quality of life benefit of mavorixafor for the indicated patient population. A cost-minimization analysis versus plerixafor may also be considered as a supplementary approach on the basis of similar mechanism of action (CXCR4 antagonism) (1, 6).
9.2 Pasientpopulasjonen som den helseøkonomiske analysen baseres på, herunder eventuelle undergrupper.	The population entering the CEM is aligned with the licensed indication for mavorixafor, being patients aged ≥12 years with a clinical diagnosis of WHIM syndrome.
9.3 Hvilken dokumentasjon skal ligge til grunn? (H2H studie, ITC, konstruert komparatorarm etc.) *Angi det som er relevant med tanke på hvilken type analyse som foreslås.*	No head-to-head study or formal indirect treatment comparison has been conducted between mavorixafor and plerixafor. Efficacy and safety data for mavorixafor are derived from the 4WHIM trial, while efficacy and safety data for plerixafor are derived from a phase III randomised crossover trial of plerixafor versus G-CSF for treatment of WHIM syndrome (2, 7). The comparative assessment is therefore based on outcomes reported in separate studies rather than a formal comparative analysis. Given the rarity of WHIM syndrome and the limited available evidence base, no suitable studies enabling a robust indirect anchored or unanchored comparison were identified.
9.4 Forventet legemiddelbudsjett i det året med størst budsjettvirkning i de første fem år.	The pricing strategy is under progress; therefore, a budget impact analysis cannot be provided at the date of the submission of request for assessment. It will be submitted together with the full HTA submission.
9.5 Forventet tidspunkt (måned og år) for levering av dokumentasjon til Direktoratet for medisinske produkter og/eller Sykehusinnkjøp HF. *Tidspunkt må oppgis*	November, 2026

10 Sykdommen og eksisterende behandling

10.1 Sykdomsbeskrivelse for aktuell indikasjon *Kort beskrivelse av sykdommens patofysiologi og klinisk presentasjon / symptombilde, eventuelt inkl. referanser*	Pathophysiology: WHIM syndrome is caused by heterozygous gain-of-function mutations in the CXCR4 gene, which encodes a chemokine receptor which guides immune cells, vital for cellular processes including cell migration, proliferation, and survival (8). The mutations prolong the activation of the receptor, causing the retention of degenerating, dysmorphic neutrophils and other leukocytes in the bone marrow (myelokathexis), leading to neutropenia (9, 10).

	Clinical presentation: WHIM syndrome is characterised by symptoms including warts, hypogammaglobulinemia, infections, and myelokathexis, although not all patients present with every component at diagnosis. While neutropenia is a nearly universal laboratory hallmark (98%), other features like cutaneous warts often do not emerge until adolescence or adulthood. The complete tetrad of warts, hypogammaglobulinemia, infections, and myelokathexis is only present in approximately 20% of patients at the time of their initial diagnosis.
10.2 Fagområde *Angi hvilket fagområde som best beskriver metoden*	Velg fagområde fra menyen: Immunologi
10.3 Kreftområde *Hvis metoden gjelder fagområdet Kreftsykdommer, angi hvilket kreftområde som er aktuelt*	Velg kreftområde fra menyen: Velg et element.
10.4 Dagens behandling *Nåværende standardbehandling i Norge, inkl. referanse*	There are no official Norwegian treatment guidelines for WHIM syndrome. According to Swedish treatment guideline, treatment of WHIM syndrome focuses primarily on preventing and managing infections. Antibiotics are used to reduce the risk of serious bacterial infections, and live vaccines are contraindicated because of the increased risk of infection. Neutropenia is treated with granulocyte colony-stimulating factor (G-CSF) to increase neutrophil counts, while antibody deficiency is managed with immunoglobulin replacement therapy (IgRT). In addition, targeted therapies that inhibit the underlying disease mechanism, an overactive CXCR4 receptor, can improve and normalize many symptoms. Associated autoimmune diseases are treated with appropriate medications according to the specific condition. The only potentially curative treatment for the immunodeficiency is haematopoietic stem cell transplantation (HSCT), which can also lead to resolution of the characteristic warts. Psychological and social support for affected children and their families is an important component of comprehensive care (11).
10.5 Prognose *Beskriv prognosen med nåværende behandlingstilbud, inkl. referanse*	Because WHIM syndrome is exceptionally rare, comprehensive long-term natural history data are limited. However, the clinical prognosis is substantially poorer than that of the general population; WHIM syndrome patients have a median estimated life expectancy of circa 55 years (12). While acute, life-threatening infections such as bacterial meningitis, sepsis, and osteomyelitis can occur in WHIM syndrome, both severe infections and malignancies, particularly HPV-induced carcinomas and EBV-related lymphomas, contribute substantially to

	morbidity and premature mortality (2, 8, 12, 13). The 40-year cancer risk is approximately 30% (2, 12, 13), with malignancies typically occurring after the third decade of life (8, 12). With modern management including Granulocyte Colony-Stimulating Factor (G-CSF), Intravenous Immunoglobulin (IVIG), and prophylactic antibiotics, infection-related mortality can be reduced, though cancer remains a major long-term concern requiring vigilant surveillance (8, 12-14). Apart from the symptomatic treatment, off-label use of plerixafor, a CXCR4 antagonist, and non-standard use of HSCT in very severe cases have been attempted in WHIM patients.
10.6 Det nye legemiddelets innplassering i behandlingsalgoritmen	Mavorixafor is expected to be used in addition to current standard of care (SoC), for patients who are 12 years or older with WHIM syndrome. SoC reflects the treatments used for the symptomatic management associated with WHIM syndrome.
10.7 Pasientgrunnlag *Beskrivelse, insidens og prevalens av pasienter omfattet av aktuell indikasjon* i Norge, inkl. referanse.* *Antall norske pasienter antatt aktuelle for behandling med legemiddelet til denne indikasjonen.* ** Hele pasientgruppen som omfattes av aktuell indikasjon skal beskrives*	The prevalence of WHIM, is 0.2/million to 1.4/million (15), resulting in a prevalence of 1-8 patients in Norway. Approximately 20% to 55% of these patients are diagnosed, leading to around <1-4 eligible patients in Norway. Assuming that 75% of these patients who are moderate or severe and will be treated, which means the entire patient group covered by mavorixafor is <1 to 3 patients (16).

11 Studiekarakteristika for relevante kliniske studier

	Studie 1	Studie 2	Studie 3
11.1 Studie-ID *Studienavn, NCT-nummer, hyperlenke*	A Dose Determination and Safety Study of X4P-001 (Mavorixafor) in Participants With Warts, Hypogammaglobulinemia,	Efficacy and Safety Study of Mavorixafor in Participants With Warts, Hypogammaglobulinemia, Infections, and	Klikk eller trykk her for å skrive inn tekst.

	Infections, and Myelokathexis (WHIM) Syndrome NCT03005327	Myelokathexis (WHIM) Syndrome NCT03995108 An open-label extension is followed after the completion of the study.	
11.2 Studietype og -design	Phase II open-label, prospective, international dose escalation study followed by an extension phase. N=8	Phase III, randomised, double-blind, placebo-controlled, multicentre study. Placebo; N=17, Mavorixafor; N=14.	Klikk eller trykk her for å skrive inn tekst.
11.3 Formål	To evaluate the safety, tolerability, and dose required to achieve a consistent increase in circulating neutrophils and lymphocytes in patients with WHIM syndrome	To evaluate the safety and efficacy of mavorixafor in patients with WHIM syndrome	Klikk eller trykk her for å skrive inn tekst.
11.4 Populasjon *Viktige inklusjons- og eksklusjonskriterier*	Key inclusion criteria(17): Participants with a clinical diagnosis of WHIM syndrome must meet all of the following criteria to be eligible for study participation: 1. Be at least 18 years of age. 2. Has signed the current approved informed consent form. 3. Has a genotype-confirmed mutation of chemokine receptor type 4 (CXCR4) consistent with WHIM syndrome. 4. Agree to use effective contraception. 5. Be willing and able to comply with this protocol.	Key inclusion criteria(18): Inclusion Criteria for the Randomized Placebo-Controlled Period: • Have signed the current approved informed consent form. Participants under 18 years of age (in the Netherlands and other applicable regions, participants under 16 years of age) will sign an approved informed assent form and must also have a signed parental/legal guardian consent. • Have a genotype-confirmed mutation of chemokine (C-X-C motif) receptor 4 (CXCR4)	Klikk eller trykk her for å skrive inn tekst.

	6. Has confirmed ANC less than or equal to (≤) 400/µL or ALC ≤650/µL or both. Key exclusion criteria: Participants with any of the following will be excluded from participation in the study: 1. Has known systemic hypersensitivity to the mavorixafor drug substance or its inactive ingredients. 2. Is pregnant or nursing. 3. Has a known history of a positive serology or viral load for human immunodeficiency virus (HIV) or a known history of acquired immunodeficiency syndrome (AIDS). 4. Has, at Screening, laboratory tests meeting one or more of the following criteria: ○ A positive antibody test for hepatitis C virus (HCV), unless documented to have no detectable viral load on 2 independent samples. ○ A positive test for hepatitis B surface	consistent with WHIM phenotype. • Agree to use a highly effective form of contraception. • Be willing and able to comply with the protocol. • Have confirmed ANC ≤400 cells/µL during screening, obtained while participant has no clinical evidence of infection. Inclusion Criteria for the Open-Label Period: • Completed the Randomized Period; or • Granted Early Release from the Randomized Period. Key Exclusion Criteria: • Has known systemic hypersensitivity to the mavorixafor drug substance, its inactive ingredients, or the placebo. • Is pregnant or breastfeeding. • Has any medical or personal condition, which in the opinion of the Investigator may potentially compromise the safety or compliance of the participant or may preclude the participant's successful completion of the clinical study.	

	antigen (HBsAg). 5. Has any medical or personal condition that, in the opinion of the Investigator, may potentially compromise the safety or compliance of the participant, or may preclude the participant's successful completion of the clinical study.		
11.5 Intervensjon (n) *Dosering, doseringsintervall, behandlingsvarighet*	**Initial treatment phase**: Patients initiated treatment with mavorixafor at 50 mg once daily orally or a higher dose, with potential escalation based on $AUC_{ANC/ALC}$ values to a maximum total daily dose of 400 mg. Patients were expected to receive treatment for 24 weeks in the initial treatment period or until development of a treatment-limiting toxicity **Extension phase**: All patients received mavorixafor; the dose did not exceed 400 mg. In the extension phase, treatment continued until mavorixafor becomes available via an alternative mechanism (for example, drug is commercially available, an expanded access programme, etc.) or until the study is terminated by the sponsor	Mavorixafor 400 mg in adults and adolescents weighing >50 kg, or 200 mg in adolescents weighing ≤50 kg (Note that the labelled dose for adolescents weighing ≤50 kg is 300mg) Dosing interval: Once daily Treatment duration: 52 weeks.	Klikk eller trykk her for å skrive inn tekst.
11.6 Komparator (n)	N/A	Comparator: Placebo	Klikk eller trykk her for å skrive inn tekst.

Dosering, doseringsintervall, behandlingsvarighet		Dosing: Matching intervention Dosing interval: Once daily Treatment duration: 52 weeks Furthermore, following completion of the placebo-controlled portion of the trial, more than 90% of the eligible participants opted to receive treatment with mavorixafor in the open-label trial extension.	
11.7 Endepunkter *Primære, sekundære og eksplorative endepunkter, herunder definisjon, målemetode og ev. tidspunkt for måling*	**Primary endpoint:** Mean value of the threshold-adjusted AUC_{ANC} and/or AUC_{ALC} collected over 24 hours (with ANC threshold of 600/µL and ALC threshold of 1000/µL) **Key exploratory endpoints:** Absolute and fold change from baseline for total WBC, ANC, ALC, AMC Annualised infection rate at each dose level compared with the 12 months prior to the study Number of warts on the hands and feet compared with baseline **Post-hoc analysis:** Time (hours) during which ANC was maintained at >500/µL or ALC was maintained at >1000/µL	**Primary endpoint:** • TAT_{ANC}, defined as time (hours) above ANC threshold of ≥500 cells per µL over a 24-hour period, assessed every 3 months for 52 weeks **Key secondary endpoints (hierarchical order):** • TAT_{ALC}, defined as time (hours) above ALC threshold of ≥ 1000/µL over a 24-hour period, assessed every 3 months for 52 weeks • Composite efficacy score calculated by summing the ranks of the total infection score and total wart change score at 52 weeks • Total wart change score at 52 weeks, based on Clinical Global Impression of Change (CGI-C) • Total infection score, combining infection	Klikk eller trykk her for å skrive inn tekst.

		frequency and severity **Other secondary endpoints:** • Annualised infection rate • Tetanus vaccine titres Quality of life (PGI-C, PGI-S, SF-36, EQ-5D-5L, and DLQI)	
11.8 Relevante subgruppeanalyser *Beskrivelse av ev. subgruppeanalyser*	N/A	Immunoglobulin (Ig) use and non-Ig use; patients with weight ≤ 50 kg and > 50 kg; Age; TAT_{ANC}; TAT_{ALC}; Sex; Geographic region; Initial randomisation dose, Genetic mutation	Klikk eller trykk her for å skrive inn tekst.
11.9 Oppfølgingstid *Hvis pågående studie, angi oppfølgingstid for data som forventes å være tilgjengelige for vurderingen hos Direktoratet for medisinske produkter samt den forventede/planlagte samlede oppfølgingstid for studien*	Median follow-up:16.5 months	Median follow-up times at the data cutoff for the mavorixafor and placebo groups reached 359 days (range 32 to 372 days) and 364 days (range 336 to 427 days), respectively (2).	Klikk eller trykk her for å skrive inn tekst.
11.10 Tidsperspektiv resultater *Pågående eller avsluttet studie? Tilgjengelige og fremtidige datakutt*	Completed. Primary completion: 14 June 2019 (analysis cutoff)	Completed. Data cutoff: 11 November 2022 Open-label extension for patients with WHIM and history of baseline warts was completed in December 2025.	Klikk eller trykk her for å skrive inn tekst.
11.11 Publikasjoner *Tittel, forfatter, tidsskrift og årstall. Ev. forventet tidspunkt for publikasjon*	Dale DC, Firkin F, Bolyard AA, Kelley M, Makaryan V, Gorelick KJ, et al. Results of a phase 2 trial of an oral CXCR4 antagonist, mavorixafor, for treatment of WHIM syndrome. Blood. 2020	Badolato R, Alsina L, Azar A, Bertrand Y, Bolyard AA, Dale D, et al. A phase 3 randomized trial of mavorixafor, a CXCR4 antagonist, for WHIM syndrome. Blood. 2024 Jul 4;144(1):35–45.	Klikk eller trykk her for å skrive inn tekst.

	Dec 24;136(26):2994–3003. doi:10.1182/blood.2020007197 PubMed PMID: 32870250; PubMed Central PMCID: PMC7770568.	doi:10.1182/blood.2023022658	

12 Igangsatte og planlagte studier	
12.1 Er det pågående eller planlagte studier for legemiddelet innenfor samme indikasjon som kan gi ytterligere informasjon i fremtiden? *Hvis ja, oppgi forventet tidspunkt*	Ja ☐ Nei ☒ Klikk eller trykk her for å skrive inn tekst.
12.2 Er det pågående eller planlagte studier for legemiddelet for andre indikasjoner?	Ja ☒ Nei ☐ The 4WARD is a phase 3, randomized, double-blind, placebo-controlled, multicenter study of mavorixafor in participants with congenital and acquired primary autoimmune and idiopathic chronic neutropenic disorders who are experiencing recurrent and/or serious infections (19).

13 Diagnostikk	
13.1 Vil bruk av legemiddelet til anmodet indikasjon kreve diagnostisk test for analyse av biomarkør? *Hvis ja, fyll ut de neste spørsmålene*	Ja ☐ Nei ☒
13.2 Er testen etablert i klinisk praksis? *Hvis ja, testes pasientene rutinemessig i dag?*	Ja ☐ Nei ☐ Hvis ja, testes pasientene rutinemessig i dag? Ja ☐ Nei ☐

13.3 Hvis det er behov for en test som ikke er etablert i klinisk praksis, beskriv behovet inkludert antatte kostnader/ressursbruk	Klikk eller trykk her for å skrive inn tekst.

14 Andre relevante opplysninger

14.1 Har dere vært i kontakt med fagpersoner (for eksempel klinikere) ved norske helseforetak om dette legemiddelet/indikasjonen? *Hvis ja, hvem har dere vært i kontakt med og hva har de bidratt med?* *(Relevant informasjon i forbindelse med rekruttering av fageksperter i Nye metoder)*	Ja ☒ Nei ☐ The company has been in contact with MD, PhD Børre Fevang at Oslo University Hospital to obtain a high-level understanding of the number of patients with WHIM syndrome in Norway. No formal consultancy activities were undertaken.
14.2 Anser leverandør at det kan være spesielle forhold ved dette legemiddelet som gjør at en innkjøpsavtale ikke kan basere seg på flat rabatt for at legemiddelet skal kunne oppfylle prioriteringskriteriene? *Hvis ja, begrunn kort.* *Hvis ja, skal eget skjema fylles ut og sendes til Sykehusinnkjøp HF samtidig med at dokumentasjon til metodevurdering sendes til Direktoratet for medisinske produkter.* *Nærmere informasjon og skjema:* Informasjon og opplæring - Sykehusinnkjøp HF	Ja ☐ Nei ☒ Klikk eller trykk her for å skrive inn tekst.
14.3 Andre relevante opplysninger?	Norgine would appreciate the opportunity to discuss the following questions with the NoMA prior to submission: • Does NoMA agree that plerixafor represents the most relevant comparator to mavorixafor in Norwegian clinical practice for patients with WHIM syndrome? • Given the rarity of WHIM syndrome and the limited available evidence base, does NoMA have any comments on the proposed approach for presenting the clinical evidence supporting the comparison between mavorixafor and plerixafor in the absence of both direct and formal indirect comparative evidence?

	• For WHIM, following extensive work, Norgine has undertaken a structured expert elicitation to assess the relationship between ANC (surrogate endpoint) and bacterial infections (clinical outcome) in patients with WHIM, and to estimate transition probabilities from short-term response to longer-term malignancy outcomes within the health economic model. What process, evidence, and rationale would NoMA expect to be presented in order to provide sufficient assurance of the appropriateness of this approach for decision making?

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