NYE METODER

Anmodning om revurdering av legemiddel i Nye metoder

Skjema for leverandører

En leverandør som ønsker en revurdering av en legemiddelindikasjon som tidligere er metodevurdert og har en beslutning i Nye metoder, skal anmode om revurdering i Nye metoder ved å fylle ut dette skjemaet.

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

En anmodning om revurdering skal gjelde samme indikasjon som den opprinnelige metodevurderingen. Hvis anmodningen gjelder en annen indikasjon, eller en undergruppe, så skal «Anmodningsskjema for vurdering av legemiddel» benyttes.

Dersom det ikke foreligger nye kliniske data, kun ny pris eller forslag om en alternativ prisavtale, er det ikke nødvendig å anmode om revurdering. Ta da direkte kontakt med Sykehusinnkjøp[1].

Nye metoder vurderer på bakgrunn av anmodningen om det er grunnlag for å gi et oppdrag om en ny metodevurdering. Anmodningen må begrunnes.

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): ☒

Dato for innsending av skjema: 24.08.2026

1 Kontaktopplysninger	
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[1] E-post til Sykehusinnkjøp HF: nyelegemidler@sykehusinnkjop.no

2 Informasjon om metoden	
2.1 ID-nummer i Nye metoder	ID2020_059
2.2 Virkestoff	Pemigatinib
2.3 Handelsnavn	Pemazyre
2.4 Indikasjon *En anmodning om revurdering skal gjelde samme indikasjon som den opprinnelige metodevurderingen. Hvis anmodningen gjelder en annen populasjon eller en undergruppe, så skal «Anmodningsskjema for vurdering av legemiddel» benyttes (se nyemetoder.no).*	Pemazyre monotherapy is indicated for the treatment of adults with locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement that have progressed after at least one prior line of systemic therapy.
2.5 Gjeldende beslutning fra Beslutningsforum for nye metoder *Oppgi dato for beslutning*	The current national decision from Beslutningsforum for Nye Metoder dates from 12 December 2022 and concluded that pemigatinib (Pemazyre) should not be introduced for the approved indication. In November 2024, Incyte submitted a reassessment request supported by additional clinical evidence. The request was considered by Bestillerforum for Nye Metoder on 10 February 2025, which decided not to commission a new method assessment. Consequently, the 12 December 2022 decision remains the current national decision.

3 Informasjon om eksisterende metodevurdering og grunnleggende forutsetninger for revurdering	
3.1 Klinisk praksis *Er beskrivelsen av norsk klinisk praksis i den opprinnelige metodevurderingen fortsatt gjeldende, herunder komparator, forutgående behandling osv.?* *Kommenter kort.*	The description of Norwegian clinical practice remains broadly applicable to the population considered in the original assessment, namely adults with locally advanced or metastatic cholangiocarcinoma harbouring an FGFR2 fusion or rearrangement whose disease has progressed following at least one prior line of systemic therapy. Since the original assessment, durvalumab in combination with gemcitabine and cisplatin has been introduced as a first-line treatment option for patients with advanced biliary tract cancer. Consequently, a proportion of patients receiving pemigatinib in current clinical practice will previously have received chemoimmunotherapy in the first-line setting. The clinical position of pemigatinib remains unchanged. There is still no established targeted treatment alternative for

	patients with FGFR2 fusion- or rearrangement-positive cholangiocarcinoma following progression on prior systemic therapy. For patients considered suitable for further treatment, fluoropyrimidine-based chemotherapy regimens such as FOLFOX or FOLFIRI may be used, whereas best supportive care remains relevant for patients who are not candidates for additional systemic therapy. Molecular diagnostics have become increasingly integrated into routine clinical practice. Current Norwegian guidance recommends molecular profiling, preferably using next-generation sequencing (NGS), in patients with advanced biliary tract cancer who are candidates for systemic treatment. This facilitates identification of patients with FGFR2 fusions or rearrangements and supports the use of precision medicines in molecularly defined subgroups. Accordingly, the present reassessment concerns the same clinical treatment setting as the original assessment. While first-line management has evolved through the introduction of chemoimmunotherapy and broader use of molecular profiling, patients with FGFR2 fusion- or rearrangement-positive cholangiocarcinoma who progress after systemic therapy continue to represent a population with substantial unmet need and limited treatment alternatives. Pemigatinib therefore retains the same clinical role as a targeted treatment option for this molecularly defined patient group
3.2 Nye data for bruken av legemiddelet til aktuell indikasjon *Beskriv kort hvorfor det er grunnlag for en ny metodevurdering av legemiddelet. Beskriv tilgjengelige nye data for legemiddelet.*	Since the original assessment, the evidence base for pemigatinib in previously treated cholangiocarcinoma with FGFR2 fusion or rearrangement has expanded substantially and now includes new prospective clinical data directly relevant to the approved treatment setting. The most important new evidence is derived from the phase III FIGHT-302 study. Although FIGHT-302 was designed as a randomized first-line trial, patients randomized to gemcitabine/cisplatin were eligible to cross over to pemigatinib following disease progression. This resulted in a prospectively collected second-line crossover population of 42 patients treated with pemigatinib within the framework of a randomized clinical trial. In this crossover population, objective response rate was 38.1%, median progression-free survival was 8.1 months and median duration of response was 6.2 months. These outcomes are highly consistent with the final results from FIGHT-202, the pivotal phase II study that formed the basis of the original assessment. The replication of treatment outcomes in an independent prospectively collected dataset strengthens confidence in the reproducibility and generalisability of pemigatinib benefit in the approved treatment setting.

	Additional integrated analyses combining FIGHT-202 and the FIGHT-302 crossover population further support the consistency of clinical outcomes across studies. In the integrated previously treated population (N=150), objective response rate was 37.3%, median progression-free survival was 7.5 months, and median overall survival was 17.5 months. These analyses increase the available evidence base and reduce uncertainty associated with small individual datasets. The evidence base has also expanded through supportive clinical and real-world evidence. A French and Italian real-world analysis including 72 patients with FGFR2-positive cholangiocarcinoma treated with pemigatinib in routine clinical practice reported outcomes consistent with those observed in the clinical trials, including an objective response rate of 45.8%, median progression-free survival of 8.7 months and median overall survival of 17.1 months. Additional real-world evidence from the United States supports the relevance of clinical trial outcomes in routine practice. The safety database has also increased substantially through continued clinical development and integrated regulatory analyses. No new safety concerns have been identified, and the overall safety profile remains consistent with the currently approved product information. Taken together, the evidence base is no longer limited to the single-arm FIGHT-202 study that formed the basis of the original Norwegian assessment. It now includes prospectively collected second-line data from the phase III FIGHT-302 study, larger integrated analyses, additional clinical evidence, real-world evidence and expanded safety follow-up. Collectively, these data directly address key uncertainties identified in the original assessment and provide a materially broader and more robust evidence base for pemigatinib in the same previously assessed indication.
3.3 Hvilken type helseøkonomisk analyse foreslår leverandøren? *Type metodevurdering* *Begrunn kort*	Incyte proposes a simplified reassessment (forenklet metodevurdering/revurdering). A new cost-utility analysis is not proposed as part of the reassessment request. The original assessment was conducted as a simplified assessment of efficacy, safety and costs. Since that assessment, the evidence base for pemigatinib has expanded substantially and now includes prospectively collected second-line data from the FIGHT-302 crossover population, integrated analyses across previously treated patients, supportive clinical evidence, real-world evidence and expanded safety follow-up. These data directly relate to the previously assessed indication and provide a materially

	broader and more mature evidence base than was available at the time of the original assessment. The primary purpose of the reassessment should therefore be to evaluate the impact of this expanded clinical evidence on the overall assessment of clinical benefit and uncertainty. The available evidence demonstrates consistent clinical activity across multiple independent datasets, including prospective clinical trial populations and routine clinical practice, and provides more mature information on response, duration of response, progression-free survival, overall survival, treatment duration and safety. The requested reassessment concerns the same patient population, treatment setting and decision problem as the original evaluation and could therefore be informed through an updated review of the available clinical evidence together with current estimates of patient numbers, treatment duration, drug costs and budget impact. Incyte therefore considers a simplified reassessment to be the most proportionate and decision-relevant approach for evaluating the new evidence available for pemigatinib in adults with locally advanced or metastatic FGFR2 fusion- or rearrangement-positive cholangiocarcinoma following prior systemic therapy. The updated evidence directly addresses key uncertainties identified in the original assessment and provides a stronger basis for reassessing the benefit, uncertainty and economic consequences of treatment in Norway.
3.4 Forventet tidspunkt (måned og år) for levering av dokumentasjon til Direktoratet for medisinske produkter *Tidspunkt må oppgis*	September 2026. Incyte expects to submit updated documentation to DMP in September 2026, should Bestillerforum decide to commission a reassessment.
3.5 Nye data for komparator *Beskriv eventuelle nye data for komparator*	No new randomized comparator data has been identified specifically for patients with previously treated FGFR2-positive cholangiocarcinoma. The comparator situation therefore remains broadly unchanged from the original Norwegian assessment. For patients fit enough for further cytotoxic therapy after first-line treatment, combination chemotherapy remains the main active treatment alternative. For patients unsuitable for further chemotherapy, active symptom control/best supportive care remains clinically relevant. The available second-line chemotherapy evidence, including FOLFOX from ABC-06, remains based on an unselected biliary tract cancer

	population and not specifically on patients with FGFR2 fusion or rearrangement. The main new evidence in this request is therefore not new comparator evidence, but new evidence for pemigatinib in the approved treatment setting. In particular, the FIGHT-302 crossover population provides prospectively collected data for pemigatinib used after progression on gemcitabine plus cisplatin and is addressed under the section on new data for the medicinal product. Real-world evidence also confirms that chemotherapy-based regimens remain relevant in treatment sequencing. In the US claims analysis, the most common baseline regimens before pemigatinib included gemcitabine plus cisplatin, FOLFOX and gemcitabine plus cisplatin plus immunotherapy. These data are descriptive and should not be interpreted as comparative efficacy data.
3.6 Øvrige forhold *Beskriv eventuelle andre forhold som er endret siden forrige metodevurdering*	Several relevant conditions have changed since the original Norwegian assessment. Norwegian clinical practice has moved further towards molecular profiling and biomarker-guided treatment selection in biliary tract cancer. Current Norwegian guidance recommends molecular profiling of tumour tissue, preferably using next-generation sequencing (NGS), for patients with inoperable or advanced biliary tract cancer who are candidates for systemic treatment. This facilitates identification of patients with actionable molecular alterations, including FGFR2 fusions or rearrangements, and supports the clinical relevance of targeted treatment approaches in molecularly defined subgroups. The first-line treatment landscape has evolved. Durvalumab in combination with gemcitabine and cisplatin has been introduced in Norway as first-line treatment for adults with inoperable or metastatic biliary tract cancer. As a result, patients eligible for pemigatinib in current clinical practice will increasingly have received chemoimmunotherapy before progression. This development changes the prior treatment context but does not remove the need for effective treatment options after progression on systemic therapy. The clinical relevance of FGFR2-positive cholangiocarcinoma as a distinct molecular subgroup has

	become more prominent. FGFR2 fusions or rearrangements define a small patient population for whom FGFR-targeted treatment is biologically and clinically relevant. However, no FGFR-targeted therapy has been introduced in the Norwegian specialist healthcare service for this population. The practical consequences of the current access situation have become increasingly visible since the original assessment. Individual Norwegian patients have sought access to Pemazyre outside the publicly funded healthcare system. This illustrates the continuing unmet medical need in this very small molecularly defined population and the consequences of the absence of an introduced FGFR-targeted treatment option in Norway. The issue has also been the subject of repeated dialogue between Incyte, Nye Metoder, Beslutningsforum and Sykehusinnkjøp, including discussions on reassessment, price offers, alternative agreement models and temporary introduction. Taken together, these developments support reassessment. Since the original assessment, Norwegian oncology practice has moved further towards molecular diagnostics and precision medicine, while patients with FGFR2 fusion- or rearrangement-positive cholangiocarcinoma continue to lack access to an introduced FGFR-targeted treatment option within the public specialist healthcare service. The current request therefore concerns a treatment whose clinical relevance has increased in the evolving precision-oncology context and whose reassessment is supported by newly available evidence in the previously assessed indication.

4 Relevansen av nye data

4.1 Beskriv hvordan de nye dataene kan bidra til at prioriteringskriteriene kan bli oppfylt. *Redegjør for de nye dataene sammenlignet med de opprinnelige resultatene som lå til grunn for gjeldende beslutning i Beslutningsforum.*	The evidence base for pemigatinib in adults with locally advanced or metastatic cholangiocarcinoma harbouring an FGFR2 fusion or rearrangement, with progression after at least one prior line of systemic therapy, has expanded substantially since the original assessment. The original assessment was conducted as a simplified method assessment of efficacy, safety and costs. At that time, the evidence base was primarily derived from FIGHT-202, a single-arm phase II study in previously treated patients. The Norwegian process identified uncertainty related to the study design, the absence of robust comparative data and uncertainty regarding survival benefit, which affected the ability to establish cost-effectiveness. Since then, the evidence base has become broader, more mature and more directly relevant to the previously assessed

	indication. The key new evidence consists of prospectively collected second-line data from the FIGHT-302 crossover population, final mature FIGHT-202 data, integrated analyses of previously treated patients, supportive clinical evidence, real-world evidence from routine clinical practice and an expanded safety database. The most important new evidence is the FIGHT-302 crossover population. FIGHT-302 was a randomized phase III study of pemigatinib versus gemcitabine plus cisplatin in previously untreated patients with advanced cholangiocarcinoma harbouring FGFR2 fusion or rearrangement. Although the randomized first-line comparison is not the basis for this reassessment request, patients in the gemcitabine/cisplatin arm were eligible to cross over to pemigatinib following disease progression. This generated a prospectively collected second-line pemigatinib population within the framework of a phase III clinical trial. In the FIGHT-302 crossover population, 42 patients received pemigatinib after progression on gemcitabine/cisplatin. Objective response rate was 38.1%, median progression-free survival was 8.08 months and median duration of response was 6.21 months. These outcomes are consistent with final FIGHT-202 results in previously treated cholangiocarcinoma with FGFR2 fusion or rearrangement, where objective response rate was 37.0%, median progression-free survival was 7.03 months, median duration of response was 9.13 months and median overall survival was 17.48 months. The consistency between FIGHT-202 and the FIGHT-302 crossover population is highly relevant to the current reassessment. It demonstrates that the clinical activity observed in FIGHT-202 has been reproduced in an independent, prospectively collected second-line dataset. This directly addresses an important source of uncertainty in the original assessment: whether the treatment outcomes observed in a single-arm phase II study were reproducible in a further clinical dataset relevant to the approved treatment setting. Integrated analyses of the previously treated cholangiocarcinoma population provide additional support for the stability of the efficacy estimates. In the integrated population combining FIGHT-202 and the FIGHT-302 crossover population (N=150), objective response rate was 37.3%, median progression-free survival was 7.52 months, median duration of response was 7.49 months and median overall survival was 17.48 months. These results are consistent with the individual studies and provide a broader basis for assessing clinical benefit, treatment duration and uncertainty in the approved indication.

Real-world evidence further supports the external validity of the clinical trial findings. In a combined French and Italian observational dataset including 72 patients with FGFR2-positive cholangiocarcinoma treated with pemigatinib in routine clinical practice, objective response rate was 45.8%, median progression-free survival was 8.7 months and median overall survival was 17.1 months. These descriptive real-world data are not intended to establish comparative effect, but they support the consistency of pemigatinib outcomes across clinical trials and routine practice.

The safety evidence base has also expanded since the original assessment. The available evidence remains consistent with the known safety profile of pemigatinib, and no new safety concerns have been identified in the updated evidence package. The expanded safety data provide a more mature basis for assessing adverse events, dose modification, treatment interruption, discontinuation and expected resource use in Norwegian clinical practice.

The new evidence is relevant to the Norwegian prioritisation criteria in the following ways.

Prioritisation criterion	**Relevance of the new evidence**
Benefit	The updated evidence supports clinically meaningful and reproducible pemigatinib activity in the previously assessed indication. Response, duration of response, progression-free survival and overall survival outcomes are consistent across FIGHT-202, the FIGHT-302 crossover population, integrated analyses and real-world evidence. This provides a stronger and more mature basis for assessing treatment benefit than was available at the time of the original decision.
Resource use	The updated evidence provides more mature information on treatment duration, progression-free survival, overall survival, dose modification, discontinuation, safety and real-world use. These data can support an updated simplified assessment of expected drug costs, healthcare resource use and budget impact in Norway.
Severity	The relevant population remains a very small molecularly defined subgroup of patients with advanced cholangiocarcinoma. The FIGHT-302 programme illustrates the rarity of this population, as more than 4,500 patients were prescreened to randomize 167 patients with FGFR2-rearranged cholangiocarcinoma. The current Norwegian access situation also demonstrates that patients in this subgroup continue to lack an introduced FGFR-targeted treatment option within the public specialist healthcare service.

The table below summarises how the new evidence addresses key uncertainties from the original Norwegian assessment.

Original uncertainty in the Norwegian assessment	New available evidence	Decision relevance
Evidence was mainly based on FIGHT-202, a single-arm phase II study, and relative effect could not be established	Prospectively collected second-line pemigatinib data from the FIGHT-302 crossover population	Demonstrates reproducibility of pemigatinib activity in an independent dataset directly relevant to the approved treatment setting.
Time-to-event outcomes were uncertain due to limited data maturity	Final FIGHT-202 data, FIGHT-302 crossover data and integrated analyses of previously treated patients	Provides a broader and more mature basis for assessing progression-free survival, overall survival, duration of response, treatment duration and uncertainty.
Clinical benefit was based on a limited dataset	FIGHT-202, FIGHT-302 crossover, integrated previously treated population, supportive clinical evidence and real-world evidence	Provides a substantially broader totality of evidence for assessing clinical benefit and uncertainty.
Generalisability to routine clinical practice was uncertain	Real-world evidence from European routine clinical practice and additional real-world evidence from the US	Supports consistency of outcomes beyond the clinical trial setting.
Safety evidence was limited by exposure and population size	Expanded clinical and regulatory safety evidence	Provides a more mature basis for assessing safety, tolerability and resource implications.

Incyte considers that the updated evidence directly addresses the key evidence limitations that informed the original Norwegian decision. The clinical benefit observed in FIGHT-202 has now been reproduced in a prospectively collected second-line population from FIGHT-302 and is supported by integrated analyses and real-world evidence. The totality of evidence provides a materially stronger basis for reassessing pemigatinib in the same previously evaluated indication and supports a new simplified assessment under the Norwegian prioritisation criteria.
