Nye metoder - Request for reassessment of medical product

A health technology developer seeking reassessment of a medicinal product/indication previously assessed by Nye metoder, should contact Nye metoder using this form, provided requirements detailed below are met.

Please send the completed form to Nye metoder by e-mail: nyemetoder@helse-sorost.no.

A request for reassessment must apply to the same population as the original assessment. If the request relates to another population or a subpopulation, then the form titled "Request for assessment of medicinal product" should be used (see nyemetoder.no).

If there is no new clinical data, a request for reassessment is not warranted. If only the cost of the new method has changed since the previous assessment, contact the Norwegian Hospital Procurement Trust (Sykehusinnkjøp HF) directly[1].

This form must be completed in its entirety. Based on the request, Nye metoder will assess whether there are grounds for commissioning a reassessment. The request must be justified.
Information about Nye metoder can be found online (nyemetoder.no). Please contact Sekretariatet for nye metoder if you have any questions.

Please note: The form will be published in its entirety.

The submitter is aware that the form will be published in its entirety (**tick**): ☑

1 Contact information

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[1]Norwegian Hospital Procurement Trust e-mail : nyelegemidler@sykehusinnkjop.no

2 Medical product overview and assessment history

Nye Metoder ID Number	ID2020_059
Active substance	Pemigatinib
Trade name	Pemazyre
Indication *A request for reassessment must apply to the same population as the original assessment. If the request relates to another population or a subpopulation, the form titled "Request for assessment of medicinal product" should be used (see 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.
Current decision from Beslutningsforum for nye metoder (Nye metoder's Decision Forum) *Date?*	Decision in the Decision Forum for New Methods (12.12.2022): Pemigatinib (Pemazyre) is not introduced as monotherapy for the treatment of adults with locally advanced or metastatic cholangiocarcinoma with fibroblast growth factor receptor 2 (FGFR2) fusion or rearrangement, with progression after at least one prior course of systemic therapy.

3 Basic prerequisites for reassessment

Clinical practice *Is the description of Norwegian clinical practice in the original assessment still applicable, including comparator, prior treatment etc.?* *Briefly describe.*	The original description of Norwegian clinical practice for treating cholangiocarcinoma remains applicable. First-line treatments include GemOx, GemCis, and GemCap regimens as described in the national treatment guidelines. Combination chemotherapy with 5-FU/oxaliplatin (FLOX or FOLFOX) has also been used in the first line. Treatment with durvalumab in combination with GemCis is also approved by Beslutningsforum as a first line treatment There is no established second-line treatment. 5-FU/irinotecan-based chemotherapy (FLIRI or FOLFIRI) can still be considered for patients in good performance status.
New data for the medicinal product *Briefly describe why there are grounds for a reassessment of the method. Describe the available new data*	Since the initial assessment, additional data from real-world studies in Europe and the US confirm the clinical benefit observed in FIGHT-202. This includes improved progression-free survival (PFS) and overall survival (OS) outcomes in broader patient populations, as demonstrated in the PEMI-BIL and PEMI-REAL studies. The FIGHT-202 final analysis also presented a median OS of 17.5 months, demonstrating the clinical benefit for patients with limited options. New safety information: Updated safety data suggests that adverse events such as hyperphosphatemia, alopecia, and retinal detachment are manageable with appropriate monitoring. Clinicians treating patients have become more experienced in managing these toxicities.
Expected date (quarter/year) for submission of documentation to Norwegian Medicines Agency *Dates must be stated*	Q1 2025
New data for the comparator *Describe any new data for the comparator*	The comparator arm in previous analyses (e.g., ABC-06 using FOLFOX) has been challenged for its relevance as it lacked FGFR2 data and focused purely on the second-line population. However, real-world evidence from studies such as PEMI-BIL suggests that Pemazyre could provide better PFS compared to historical chemotherapy outcomes for patients with FGFR2-positive cholangiocarcinoma. The final data from FIGHT-202 confirms previously reported efficacy with a median OS of 17.5 months and an ORR of 37%. The extended follow-up shows consistent results with the initial analysis, providing further confidence in the drug's effectiveness for patients with FGFR2 fusions. The new data reinforces the unmet medical need for treating FGFR2-positive cholangiocarcinoma and provides evidence that Pemazyre is effective in a population with very few alternatives. Its use could lead to better survival outcomes, especially for patients with limited remaining options. Given the rarity of the disease, this data should support the prioritisation of Pemazyre as a reimbursable treatment.

Other conditions *Describe any other conditions that have changed since the previous assessment*	Discussions with Nordic oncologists suggest that there is a high unmet need for targeted therapies like Pemazyre, as many patients are currently not eligible for second-line chemotherapy due to the side effects associated with traditional treatments. The newly presented data emphasizes the need for a targeted treatment option with a manageable toxicity profile. European guidelines (ESMO) states that patients with an identified genetic alteration are recommended to get a matching targeted therapy. For patients with identified FGFR2-fusions, pemigatinib, infigratinib and futibatinib are recommended. However, Helsinn Birex Pharmaceuticals Ltd withdrew its application for a marketing authorisation of infigratinib for the treatment of cholangiocarcinoma in EU on the 11th of October 2022. In September 2024 futibatinib was canceled by Bestillerforum. To add to the above, we reached out to one of the leading health care professionals in this area and asked for his comment on potential bias in the overall survival outcome for the pemigatinib treated population in the FIGHT-202 study. This health care professional is universally recognized as a leading expert in the field and was one of the clinical investigators in the ABC-06 trial.

4 Relevance of new data

New data *Give an account of the new data compared to the original results that formed the basis for the current decision by Beslutningsforum for Nye metoder (Nye metoder's Decision Forum)* *Describe how the new data can help meet the prioritisation criteria.*	EMAs considerations on evidence from single-arm trials conclude that single arm clinical trials (SAT) can provide pivotal evidence for drug efficacy under specific conditions, but they carry a high risk of bias due to the absence of a control group. Therefore, careful trial design, stringent statistical analysis, and adherence to regulatory standards are essential to ensure SATs provide valid, interpretable results for regulatory decisions. The paper highlights that SAT findings should be interpreted cautiously and supplemented by additional evidence whenever possible. Incyte claims that the newly published studies provide strong RWE that supplements the FIGHT-202 SAT findings on pemigatinib. By addressing real-world outcomes, diverse patient populations, comprehensive safety profiles, and rigorous statistical methods, these studies fulfill EMA's guidance on supplementing SAT data to mitigate bias and validate efficacy in broader clinical practice. Together, they provide the additional evidence that the EMA recommends ensuring SAT findings are reliable, interpretable, and applicable to the real-world treatment landscape, making a compelling case for pemigatinib's role in managing FGFR2-positive CCA.