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Tyrosine Kinase Inhibitors in myeloid/lymphoid neoplasms with FGFR1 rearrangement: A Systematic Literature Review

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Abstract

Objective: To review evidence on the efficacy and safety of tyrosine kinase inhibitors (TKIs) for myeloid/lymphoid neoplasms with FGFR1 rearrangement (MLN-FGFR1).

Methods: Medline, Embase, Cochrane Central Register of Controlled Trials (CENTRAL) databases, clinical trial registries, and major oncology and haematology conference proceedings were systematically searched. Data were collected through November 14, 2025.

Results: Among 229 identified records, 15 studies met the inclusion criteria: four single-arm clinical trials, one observational study, and ten case reports or case series. No randomized controlled trials were identified. In total, outcomes were reported for 85 patients. Pemigatinib demonstrated the highest clinical activity, with rapid and durable responses and a safety profile consistent with FGFR inhibitors. Olverembatinib induced high rates of complete remission or complete haematologic remission and sustained disease control in most cases. However, interpretation is limited by the small cohort and short follow-up. Treatment with olverembatinib was generally well tolerated. Evidence for other agents was confined to a small observational cohort and individual reports, showing heterogeneous, mostly short-lived responses, with long-term remissions occurring mainly after allogeneic haematopoietic stem cell transplantation (HSCT). Overall, selective FGFR1 inhibitors, particularly pemigatinib, demonstrated superior therapeutic activity, whereas data for multi-target TKIs remained sparse and inconsistent. Methodological heterogeneity and population diversity precluded quantitative synthesis.

Conclusion: Current evidence supports pemigatinib as the TKI with the most consistent clinical activity in MLN-FGFR1, while data for alternative agents remain limited and heterogeneous. Further prospective studies are required to inform treatment sequencing and long-term management.

INTRODUCTION

Myeloid/lymphoid neoplasms with FGFR1 rearrangement (MLN-FGFR1), historically known as 8p11 myeloproliferative syndrome (EMS) or stem cell leukaemia/lymphoma (SCLL), represent a rare subgroup within the World Health Organization (WHO) category of myeloid and lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions (MLN-eo-TK).[1–7] The disease is driven by structural abnormalities involving 8p11, which generate fusion genes between fibroblast growth factor receptor 1 (FGFR1) and multiple partner genes, most commonly ZMYM2, BCR, CNTRL, FGFR1OP, or CEP110.[1,3,6,8–10] The resulting fusion proteins retain the active FGFR1 kinase domain and acquire oligomerisation motifs from the partner gene, leading to constitutive activation of key proliferative signalling pathways.[6,7,10,11]

MLN-FGFR1 is a stem cell–derived neoplasm, as evidenced by shared 8p11 rearrangements across myeloid and lymphoid lineages.[2–4,6] Clinically, it spans chronic myeloproliferative or myelodysplastic/myeloproliferative disease and diverse acute leukaemia, including acute myeloid leukaemia (AML), T- or B-cell acute lymphoblastic leukaemia/lymphoma (ALL/LBL), and mixed-phenotype acute leukaemia (MPAL).[2–5,7–9] Extramedullary involvement is frequent, often presenting as T-lymphoblastic lymphoma, while peripheral blood commonly shows eosinophilia with variable neutrophilia or monocytosis, and bone marrow demonstrates myeloproliferative neoplasm (MPN)-like changes or blast infiltration depending on the disease phase.[3,5,9,12] The clinical course is aggressive, with transformation to acute leukaemia typically occurring within one to two years.[9,12]

MLN-FGFR1 constitutes fewer than 1% of myeloproliferative neoplasms, with an estimated incidence of approximately 0.035 cases per 100,000 individuals annually.[3,5] The disease affects all age groups, shows a moderate male predominance, and the median age at diagnosis falls in the third or fourth decade of life.[6,9] Globally, only around 100–110 cases have been reported.[6,13]

Diagnosis requires identification of an FGFR1 rearrangement. Conventional cytogenetics detects abnormalities involving 8p11–12, while break-apart fluorescence in situ hybridisation (FISH), polymerase chain reaction (PCR)/reverse transcription PCR (RT-PCR), or next-generation sequencing (NGS) confirm the fusion and enable recognition of cryptic events.[3–5,7,8,12] WHO diagnostic criteria for MLN-eo-TK require the presence of a myeloid and/or lymphoid neoplasm, often accompanied by eosinophilia, together with a documented FGFR1 rearrangement.[12]

Conventional chemotherapy offers only transient responses, and long-term remissions are typically achieved exclusively with allogeneic haematopoietic stem cell transplantation (HSCT), which is recommended early in the disease course.[3–7] Targeted FGFR-directed therapies have markedly expanded treatment options. Pemigatinib, a selective FGFR1–3 inhibitor, is the first FDA-approved treatment for relapsed or refractory MLN-FGFR1.[3,12,14] Additional TKIs, including ponatinib, midostaurin, futibatinib, dovitinib, and AZD4547, show activity in preclinical models and selected clinical cases, although responses remain variable.[1–4,8–11,15–19]

To date, no systematic review dedicated specifically to TKIs in MLN-FGFR1 has been published. Existing literature consists mainly of non-systematic or narrative reviews focusing on 8p11 myeloproliferative syndrome/EMS, broader FGFR-rearranged malignancies, or individual agents, without the use of systematic methodology or comparative assessment of TKIs efficacy and safety in MLN-FGFR1. In the absence of randomized trials and with an increasing number of real-world reports describing outcomes with different FGFR1-targeted therapies, a structured synthesis of current evidence is warranted.

This systematic review integrates data from eligible clinical and observational studies and case reports to evaluate the effectiveness, safety, and clinical application of TKIs in MLN-FGFR1. By organising and critically appraising the available evidence, it aims to clarify the role of TKIs in disease management and inform treatment decisions in this ultra-rare malignancy.

METHODS

This systematic review was conducted according to the updated guidelines for reporting systematic reviews (PRISMA 2020).[20]

Search strategy and selection criteria

A systematic search of Medline, Embase, and the Cochrane Central Register of Controlled Trials (CENTRAL) was conducted on 14 November 2025, using terms related to the population (8p11 myeloproliferative syndrome, myeloid/lymphoid neoplasm, FGFR1 rearrangement) and intervention (tyrosine kinase inhibitor), with no time restriction. A language restriction was applied, and publications in English or Polish were included. Trial registries (ClinicalTrials.gov, ClinicalTrialsRegister.eu, and the International Clinical Trials Registry Platform), abstracts from recent (2024–2025) annual meetings of the American Society of Hematology (ASH), American Society of Clinical Oncology (ASCO), European Hematology Association (EHA), American Association for Cancer Research (AACR), and Society of Hematologic Oncology (SOHO), and the reference lists of included studies were also hand-searched to identify additional relevant evidence. The detailed search strategies are presented in Supplementary Tables 1–2.

Inclusion and exclusion criteria were defined using the PICO framework. Studies were included if they involved patients with MLN-FGFR1 treated with TKIs and reported efficacy, safety, or quality-of-life outcomes. Eligible designs comprised clinical trials, observational studies, systematic literature reviews (SLRs), and case reports. All studies were required to be published in English or Polish. Studies in healthy volunteers or in patients with other eosinophilic disorders receiving non-TKI interventions, non-systematic reviews, commentaries, letters, personal opinions, and studies that did not report outcome data were excluded. The PICOS criteria are summarised in Supplementary Table 3. The search strategy was designed to capture both experimental and real-world evidence on the efficacy and safety of TKIs in patients with MLN-FGFR1.

Study selection, quality assessment and data extraction
Two authors (J.W.G. and K.J.Z.) independently screened titles and abstracts for eligibility using the predefined selection criteria. Studies deemed potentially relevant were then assessed in full text by both reviewers. Any disagreement was resolved by discussion and consensus. Risk of bias assessment was restricted to full-text clinical studies. Case reports and studies available only in abstract form were excluded from formal appraisal because the information provided was insufficient for reliable evaluation. Full-text studies were independently assessed by two reviewers (J.W.G. and K.J.Z.) using the National Heart, Lung, and Blood Institute (NHLBI) Study Quality Assessment Tools.[21]

Data extraction was performed by one author (J.W.G.) and independently checked by a second author (A.A.M.) for accuracy. Extracted data included first author, publication year, study name, trial identifier, study design, intervention, number of participants, sex, patient age, confirmation of FGFR1 rearrangement, race, diagnosis, number of prior lines of therapy (before TKI), history of HSCT, and clinical outcomes such as treatment response, progression-free survival (PFS), overall survival (OS), and safety.

RESULTS

Search results and characteristics of included studies
A total of 229 records were identified through the initial database search. After removal of duplicates and preliminary screening of titles and abstracts, 46

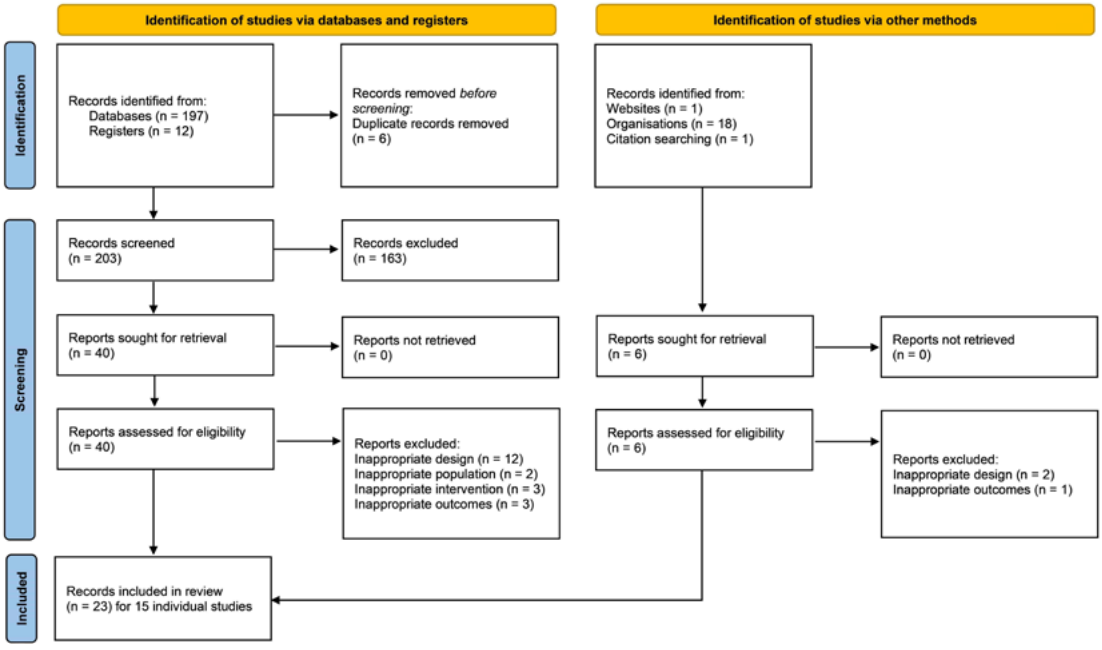

Figure 1. PRISMA diagram showing the study selection process, adapted from Page et al. [20]

Table 1. Summarized Characteristics of Included Studies Evaluating TKIs

Study and sources	Study ID number	Study Design	TKI	MLN-FGFR1 Patients' characteristic							
				N	Gender n (%)	Age, years	Initial diagnosis	Confirmed FGFR1	Race n (%)	Prior lines of therapy n (%)^^	Prior SCT/ BMT n (%)
Clinical trials											
FIGHT-203 [2,4,22,23]	NCT: NCT03011372 EudraCT: 2016-002596-10	P2, OL, SA, MC Study status: Completed DCO: November 27, 2024 Median follow-up (range): 62.9 months (29.2-90.2)	Pemigatinib 13.5 mg PO QD 2 wks on/1 wk off or continuous	47#	F: 25 (53) M: 22 (47)	Median (range): 62 (23-78)	MLN	n (%): 45 (96)	White: 30 (64) Asian: 4 (9) Black: 4 (9) Other^: 9 (19)	0: 6 (13) 1: 24 (51) 2: 8 (17) 3+: 9 (19)	3 (6)
FIGHT-101 [2,24,25]	NCT: NCT02393248	P1/2, OL, DE Study status: Terminated DCO: October 12, 2016 Follow-up: NR	Pemigatinib 1-20 mg PO QD (9 mg QD in patient with MLN-FGFR1)	1**	NR	NR	MPN	Yes	NR	NR	NR
SZ-FGFR1 [27,28]	NCT: NCT05521204	P2, OL, SA, MC Study status: Recruiting DCO: July 30, 2025 Median follow-up (range): 9.5 (2-36) months	Olverembatinib 40 mg PO QOD CP: monotherapy BP: with AML/ ALL regimens	16##	F: 9 (56) M: 7 (44)	Median: 44	Nd or Rp MLN-FGFR1 CP: 4 (25) BP:12 (75)	NR	NR	NR	NR
TAS-120-202 [29,30]	NCT: NCT04189445 EudraCT: 2019-004084-49	P2, OL, MC, SA with 3 distinct cohorts* Study status: Terminated Follow-up: NR	Futibatinib 20 mg PO QD; continuous 28-day cycles*	0***	N/A	N/A	N/A	N/A	N/A	N/A	N/A
Observational study											
PON-RS [31,32]	N/A	Retrospective survey Median follow-up (range): 21 months (9-38)	Ponatinib 30 mg QD (n=2) 45 mg QD (n=5)	7	F: 2 (29) M: 5 (71)	Median (range): 52 (48-74)	MLN-eo	n (%): 7 (100)	NR	1: 7 (100)	0 (0)
Case series and Case reports											
Hernández-Boluda 2022 [7]	N/A	Retrospective case series (22 cases) Follow-up median (range): 7.0 months (2.9-34.4) from diagnosis to transplant 4.1 years from transplant	Dasatinib + chemotherapy	1	NR	41	MPN	Yes	NR	1: 1 (100)	0 (0)
			Pemigatinib + lymphocyte donor infusions	1	NR	63	MPN + AML (Granulocytic sarcoma)	Yes	NR	1: 1 (100)	0 (0)
			Ponatinib	1	NR	39	MPN	Yes	NR	0: 1 (100)	0 (0)
			Ponatinib	1	NR	65	MPN + T-ALL	Yes	NR	1: 1 (100)	0 (0)
Strati 2018 [13]	N/A	Retrospective case series (17 cases) Median follow-up (range): 11 (1-75) months	Ponatinib	1	NR	NR	AML	Yes	NR	0: 1 (100)	1 (100)
				1	NR	NR	MPN	Yes	NR	0: 1 (100)	0 (0)
Barnes 2020 [33]	N/A	Case report (2 cases) Follow-up: NA	DA 7+3 with sorafenib 400 mg BID on days 10-19	1	M	58	de novo AML	Yes	NR	0: 1 (100)	0 (0)
Huang 2025 [1]	N/A	Case report Follow-up: ~38 months	Ponatinib 30 mg + hyper-CVAD Pemigatinib 13.5 mg daily (days 1–14 of 21)	1	M	36	MLN	Yes	NR	0: 1 (100)	0 (0)

Table 1. Summarized Characteristics of Included Studies Evaluating TKIs

Study and sources	Study ID number	Study Design	TKI	MLN-FGFR1 Patients' characteristic							
				N	Gender n (%)	Age, years	Initial diagnosis	Confirmed FGFR1	Race n (%)	Prior lines of therapy n (%)^^	Prior SCT/ BMT n (%)
Chen 2023[34]	N/A	Case report Follow-up: 15 months	2L: imatinib + FLU 3L: imatinib + CLA 4L: futibatinib 5L: futibatinib, ponatinib^^^	1	M	56	T-LBL with progression to AML	Yes	African American	1: 1 (100)	0 (0)
Guo 2024[35]	N/A	Case report Follow-up: ~9 months	Dasatinib + CHOPE	1	F	62	AML	Yes	NR	0: 1 (100)	0 (0)
Ito 2025[36]	N/A	Case report Follow-up: 2.5 years	Pemigatinib	1	F	67	MLN (initial diagnosis Richter syndrome)	Yes	NR	2: 1 (100)	0 (0)
Lv 2018[37]	N/A	Case report Follow-up: >14 months	Imatinib with daunorubicin + dexamethasone + cytarabine + vindesine	1	M	9	EMS	Yes	NR	0: 1 (100)	BMT: 1 (100)
Kasbekar 2020[10]	N/A	Case report Follow-up: >18 months	Futibatinib 20 mg PO QD	1	M	55	MLN	Yes	NR	0: 1 (100)	0 (0)
Wehrli 2017[38]	N/A	Case report Follow-up: ~10 years, including ~4,5 years on TKI	1L, 3L: imatinib 100 mg QD increased to 400 mg QD, then tapered 4L: dasatinib 50 mg PO QOD	1	F	64	EMS with progression to AML	Yes	NR	0: 1 (100)	0 (0)

Abbreviations: 2L – second-line treatment; 3L – third-line treatment; 4L – fourth-line treatment; 5L – fifth-line treatment; ALL – acute lymphoblastic leukaemia; AML – acute myeloid leukaemia; BID – twice daily;` BMT – bone marrow transplantation; BP – blast phase; CHOPE – vindesine 2 mg/m² day 1 + cyclophosphamide 750 mg/m² day 1 + liposomal doxorubicin 20 mg/m² day 1 + etoposide 60 mg/m² days 1-3 + prednisone 1 mg/kg days 1-5; CLA – cladribine; CP – chronic phase; DA 7+3 – cytarabine 100 mg/m² days 1-7 + daunorubicin 60 mg/m² days 3–5; DCO – data cutoff; DE – dose-escalation; EMS – 8p11 myeloproliferative syndrome; eo – eosinophilia; EudraCT – European Union Drug Regulating Authorities Clinical Trials Database (Number); F – female; FGFR1 – fibroblast growth receptor factor 1; FLU – fludarabine; hyper-CVAD – hyperfractionated cyclophosphamide, vincristine, doxorubicin, and dexamethasone; SCT – stem cell transplantation; M – male; MC – multicentre; MLN – myeloid/lymphoid neoplasm; MPN – myeloproliferative neoplasm; n – number; N/A – not applicable; NCT – National Clinical Trial (Number); Nd – newly diagnosed; NR – not reported; OL – open label; QD – once per day; QOD – every other day; P2 – phase 2; PO – orally; Rp – relapsed; SA – single arm; T-ALL – T-cell acute lymphoblastic lymphoma; T-LBL – T-lymphoblastic leukaemia; wk – week

Notes:

*no difference in the treatment regimen between the cohorts

**among 17 patients in part 2 only one patient had MLN-FGFR1

***patients diagnosed with MLN-FGFR1 were to be included in Cohort C; the study was terminated, and no data is available for Cohort C

^including missing

^^before the first TKI

^^^after two months futibatinib was discontinued and replaced by cytarabine

#45 patients were included in the efficacy analysis and 47 in the safety analysis

##overall, 17 patients were included

publications were selected for full-text assessment. Ultimately, 15 studies (23 records) describing 85 patients with MLN-FGFR1 were included in the final analysis. The PRISMA flow diagram illustrating the selection process is presented in Figure 1.

No randomized controlled trials or published systematic reviews were identified. Four single-arm clinical studies were included: two available as full-text publications.[2,4,22-26], one reported exclusively as a conference abstract[27,28], and one registered clinical trial that was terminated prematurely and did not provide results for the population of interest.[29,30] Both full-text studies were assessed as having a moderate risk of bias. Most methodological domains were fulfilled, with limitations related to non-blinded outcome assessment and the absence of statistical testing for pre–post changes. The detailed assessment is provided in Supplementary Table 4. In addition, one retrospective observational study available as an abstract only[31,32] and ten case series and case reports[1,7,10,13,33–38] were included.

The overall sample showed an approximately balanced sex distribution (about 1:1). The age of participants across the included studies ranged from 9 to 78 years. Most studies enrolled adult patients; only one case report described a paediatric patient. In the majority of cases, HSCT had not been performed prior to the initiation of TKI therapy. Follow-up duration ranged from 14 months to 9 years. A summary of the included studies and corresponding patient characteristics is provided in Table 1.

Owing to substantial methodological heterogeneity and the limited quality of available evidence, quantitative synthesis was deemed inappropriate, and findings were summarised narratively.

Qualitative synthesis of the study results

Pemigatinib and olverembatinib were the only TKIs evaluated in prospective clinical studies. In the FIGHT-203 trial, pemigatinib demonstrated substantial antineoplastic activity, with complete response (CR) achieved in 74% and 69% of patients according to the Central Review Committee (CRC) and Investigator assessment (IA), respectively. A complete cytogenetic response (CCyR) was achieved in 73% of treated individuals based on both assessments. The median time to CR was 1.5 months, and the median durations of CR and OS were not reached after a median follow-up of 62.9 months. PFS at 12 and 24 months was 78% and 70%, while OS rates at the same time points were 79% and 72%, respectively. Treatment-emergent adverse events were consistent with the known safety profile of FGFR inhibitors, with hyperphosphataemia as the most frequent non-haematologic event and anaemia as the predominant haematologic toxicity. Six treatment-related deaths were reported.[4] Additional clinical evidence includes a case from the FIGHT-201 programme describing a sustained CCyR lasting 3.5 months at the cutoff data.[24] and three case reports documenting rapid improvement, limited benefit or rapid progression despite pemigatinib initiation.[1,7,36]

Olverembatinib showed high activity in the SZ-FGFR1 phase 2 study, in which 14 of 16 evaluated patients achieved complete remission or complete haematologic remission, including early cytogenetic or molecular responses in selected cases. After a median follow-up of 9.5 months, eleven patients remained alive without evidence of disease. Among the five patients who proceeded to HSCT, four achieved complete molecular remission, while toxicity remained manageable.[27] Together, these two prospective datasets support robust clinical activity of selective FGFR1 inhibition in MLN-FGFR1, particularly in patients treated before progression to advanced disease.
For other TKIs, evidence is limited to case reports and very small observational cohorts, which constrains the reliability of treatment effect estimates. In the retrospective PON-RS study, ponatinib predominantly induced transient haematologic responses, with durable remissions mainly achieved following subsequent allogeneic HSCT.[30,31] Additional isolated reports describe variable outcomes, ranging from CR/CCyR prior to HSCT to rapid progression despite frontline ponatinib therapy.[1,7,10] Imatinib and dasatinib produced inconsistent results, occasionally inducing temporary haematologic or clinical improvement, but progression to AML or relapse was frequent.[34,37,38] Futibatinib showed mixed activity, from lack of response in heavily pretreated AML-transformed disease.[34] to a sustained cytogenetic and haematologic remission exceeding 18 months in a single case with chronic-phase presentation[10] Sorafenib yielded minimal residual disease–negative remission in one reported patient, but relapse occurred after transplant, followed by death due to septic shock.[33] It should be underscored that for dasatinib, sorafenib, imatinib, and futibatinib, all available efficacy and safety information comes solely from case reports, as no observational or experimental studies were identified.

Overall, evidence for TKIs other than pemigatinib and olverembatinib remains sparse and derived largely from individual clinical experiences. Observed benefits with multi-target TKIs appear limited and short-lived and are strongly influenced by disease phase and subsequent HSCT. A structured overview of results is presented in Table 2.

Table 2. Summarized Characteristics of Included Studies Evaluating TKIs

TKI	Study	N	Clinical outcomes		
			Response to TKI treatment	Time-to-event outcomes for TKI treatment	TKI safety
Pemigatinib	FIGHT-203[4]	47	Central Review Committee assessment: · CR: 74% (31/42 patients), including 23 patients (96% of 24) in chronic-phase disease and 8 patients (44% of 18) in blast-phase disease · ORR: 81% (34/42 patients) · PR: 7% (3/42 patients) · SD: 14% (6/42 patients) · PD: 0 patients · CCyR: 73% (33/45 patients) · PCyR: 16% (7/45 patients) Investigator assessment: · CR: 69% (31/45 patients) · ORR: 78% (35/45 patients) · PR: 9% (4/45 patients) · SD: 20% (9/45 patients) · PD: 2% (1/45 patients) · CCyR: 73% (33/45 patients) · PCyR: 9% (4/45 patients)	PFS: median 73.9 months (95% CI 29.2–not reached) Estimated PFS: 78% at 12 months and 70% at 24 months OS: not reached Estimated OS: 79% at 12 months and 72% at 24 months Median time to CR assessed by Central Review Committee was 1.5 months. Median duration of CR was not reached. CR was maintained for ≥12 months in 18 of 31 patients	In the safety population, haematologic AEs occurred in 45%, with anemia being the most frequent (reported by 34% of patients overall; grade ≥3 reported by 17% of patients). Non-haematologic AEs were observed in all patients; hyperphosphatemia was most common (77%) and majority of the cases were grade <3. Stomatitis was the most common grade ≥3 treatment-related AEs (17%). Six deaths were attributed to TEAEs (myocardial infarction, acute kidney injury, multiorgan dysfunction, endocarditis, infection).
	FIGHT-101 [24]	1	CCyR	PFS: no progression reported; ≥3,5 months OS: N/A; ≥3,5 months; patient alive at last follow-up	For pemigatinib not reported
	Ito 2025 [36]	1	PD after pemigatinib (as 3rd line) Progressive disease that was refractory to treatment, continued, and the patient died 4 months after relapse	PFS: N/A; progression occurred within one month after pemigatinib initiation OS: ~30 months (from diagnosis to death)	Rapid disease flare/progression on pemigatinib, no specific drug-related toxicities reported
	Huang 2025[1]	1	Rapid molecular response MRD negativity achieved prior to HSCT	PFS: ~400 days OS: ~38 months (patient remained in remission 2 years after second HSCT)	Pemigatinib was poorly tolerated in the maintenance setting. Reported AEs included rash, diarrhea, ileitis and hand-foot syndrome. Treatment was permanently discontinued due to toxicity after second HSCT.
	Hernández-Boluda 2022[7]	1	CR and CCyR were achieved with donor lymphocyte infusions and pemigatinib treatment At the time of transplant the patient remained in a state of active disease	PFS: NR OS: NR At 37 months from relapse, the patient was alive	For pemigatinib not reported
Olverembatinib	SZ-FGFR1[27]	16	Overall response: · CRm or CHeRm: 88% (14/16 patients) · PR: 13% (2/16 patients) Best response: · complete molecular responses: 31% (5/16 patients) · CCyR: 19% (3/16 patients) · partial cytogenetic response: 6% (1/16 patient) · CRm or CHeRm: 44% (7/16 patients)	PFS: N/A; 11 (69%) patients without detectable disease after median follow-up of 9.5 months OS: N/A; 11 (69%) patients alive after median follow-up of 9.5 months	Olverembatinib was generally well tolerated. AEs occurred in 8 (50%) patients treated with monotherapy. Grade ≥3 AEs occurred in 5 (31%) patients, including grade 4 neutropenia, grade 3 thrombocytopenia, grade 3 hypertension and grade 3 cerebral infarction.
Ponatinib	PON-RS[31,32]	7	Transient partial haematologic responses: 86% (6/7 patients) Partial cytogenetic response: 14% (1/7 patient) PD and death: 14% (1/7 patient)	PFS: NR OS: NR Five patients who proceeded to allogeneic SCT achieved durable complete molecular remission and remained alive for a median of 15 months after allogenic SCT	For ponatinib not reported
	Hernández-Boluda 2022[7]	2	Patient 1: CCyR after ponatinib (as 1st line) Patient 2: active disease after ponatinib (as 2nd line)	Patient 1: PFS: NR; OS: NR Patient 2: PFS: NR; OS: NR	For ponatinib not reported
	Strati 2018[13]	2	Patient 1: PD after ponatinib (as 1st line) Patient 2: PRm after ponatinib (as 1st line)	Patient 1: PFS>2 months and OS >9 months Patient 2: PFS and OS >9 months	For ponatinib not reported
	Huang 2025[1]	1	CR followed by loss of molecular response prior to HSCT (treatment switched to pemigatinib)	PFS: ~100 days OS: ~38 months (patient remained in remission 2 years after second HSCT)	For ponatinib not reported
	Chen 2023[34]	1	No haematologic or cytogenetic remission was achieved after futibatinib with ponatinib (as 5th line)	PFS: N/A; no improvement after treatment OS: ~15 months (death 2 months after 6th line of treatment)	For ponatinib not reported

Table 2. Summarized Characteristics of Included Studies Evaluating TKIs

TKI	Study	N	Clinical outcomes		
			Response to TKI treatment	Time-to-event outcomes for TKI treatment	TKI safety
Imatinib	Chen 2023[34]	1	CRm – fludarabine-based induction with imatinib (as 2nd line) No haematologic or cytogenetic remission was achieved after cladribine with imatinib (as 3rd line)	PFS: ~3 month at imatinib + fludarabine, from 4 month imatinib + cladribine – no improvement after treatment OS: ~15 months (death 2 months after 6th line of treatment)	For imatinib not reported
	Lv 2018[37]	1	Remission with complete resolution of lymphadenopathy and hepatosplenomegaly after imatinib with chemotherapy	PFS: no progression reported; ≥14 months OS: N/A; ≥14 months; patient alive at last follow-up	For imatinib not reported
	Wehrli 2017[38]	1	SD after imatinib (as 1st line) Reduction of eosinophils by half and persisted cytopenias after imatinib (as 3rd line). Treatment was then switched to dasatinib.	PFS: ~3 years OS: ~10 years (from diagnosis to death)	For imatinib not reported
Dasatinib	Guo 2024[35]	1	PMRm with clinical and haematologic improvement	PFS: ~3 months (remission April 2021, relapse early July 2021) OS: ~9 months (diagnosis November 2020, death August 2021)	For dasatinib not reported
	Hernández-Boluda 2022[7]	1	CR after dasatinib with chemotherapy (as 2nd line)	PFS: NR OS: NR	For dasatinib not reported
	Wehrli 2017[38]	1	PMRm was achieved after dasatinib (as 4th line)	PFS: ~9 months OS: ~10 years (from diagnosis to death)	Pleural effusions on dasatinib requiring dose reduction and discontinuation
Futibatinib	Chen 2023[34]	1	No haematologic or cytogenetic remission was achieved after futibatinib with or without ponatinib (as 4th-5th line)	PFS: N/A, no improvement after treatment OS: ~15 months (death 2 month after 6 line of treatment)	Futibatinib was stopped after two months owing to severe neutropenia
	Kasbekar 2020[10]	1	CCyRm and CHeRm	PFS: N/A; no progression reported; sustained remission >18 months OS: N/A; ongoing; patient alive at last follow-up	Hyperphosphatemia (grade unspecified), grade 1 dry pruritic skin, grade 2 bullous rash requiring temporary treatment interruption and dose reduction
Sorafenib	Barnes 2020[33]	1	MRD-negative CRm	PFS: ~5 months (CRm, then relapse at 5 months) OS: ~6 months (death shortly after relapse)	For sorafenib not reported

Abbreviations: AE – adverse event; CCyR – complete cytogenetic response; CCyRm – complete cytogenetic remission; CHeRm – complete haematologic remission; CR – complete response; CRm – complete remission; FLAG-IDA – fludarabine, high-dose cytosine arabinoside, idarubicin, and granulocyte colony-stimulating factor; N/A – not applicable; NR – not reported; ORR – overall response rate; OS – overall survival; PCyR – partial cytogenetic response; PD – progressive disease; PFS – progression-free survival; PMRm – partial metabolic remission; PR – partial response; PRm – partial remission; SCT – stem cell transplantation; SD – stable disease; TEAE – treatment-emergent adverse events

DISCUSSION

This systematic review indicates that among currently available TKIs, pemigatinib and olverembatinib are the only agents supported by prospective clinical evidence in MLN-FGFR1. In FIGHT-203, pemigatinib produced high rates of CR, CCyR, and ORR, with durable responses and a toxicity profile consistent with FGFR inhibitors.[4] Additional case-based evidence, including the FIGHT-201 programme, confirms its biological activity.[24] but also illustrates the possibility of primary resistance in individual patients[7,36] Olverembatinib achieved very high response rates in a small phase 2 population, with the deepest molecular responses observed in patients proceeding to HSCT.[27] By contrast, ponatinib, imatinib, dasatinib, futibatinib, and sorafenib yielded variable and generally short-lived responses, with long-term remissions occurring predominantly after allogeneic HSCT rather than as a direct consequence of TKI therapy. Collectively, these findings support a therapeutic advantage of selective FGFR1 inhibition over multi-target TKIs in MLN-FGFR1. The present results are consistent with previous descriptive analyses. Earlier reports have characterised MLN-FGFR1 as an aggressive and biologically heterogeneous malignancy with poor responsiveness to conventional chemotherapy and a high risk of early transformation.[8] Early clinical experience with selective FGFR inhibitors demonstrated that profound haematologic, cytogenetic, and molecular remissions are achievable, in contrast to the transient activity historically observed with multi-target TKIs.[39] Regulatory assessments likewise indicate that pemigatinib yields higher response rates in chronic-phase disease than in blast phase, suggesting a phase-dependent therapeutic effect.[14] Expert commentaries interpret these data as a substantial improvement over historical strategies and emphasise the depth of response attainable with FGFR1-selective inhibition. At the same time, allo-HSCT remains the only intervention with well-established curative potential, positioning selective TKIs primarily as early-line therapy and as a bridge to transplantation.[40] Taken together, published evidence aligns with our findings and supports pemigatinib as the TKI with the most consistent clinical benefit, particularly in chronic-phase MLN-FGFR1.

From both clinical and policy perspectives, available data support pemigatinib as the preferred targeted therapy for adults with MLN-FGFR1. Its FDA approval for relapsed or refractory disease and inclusion in contemporary National Comprehensive Cancer Network guidelines underscore its central therapeutic role.[14,41] The high frequency of deep responses in chronic-phase disease supports its use as first-line treatment and as a bridge to HSCT. Olverembatinib shows promising activity in early-phase studies.[14], but confirmatory data are needed. Other TKIs may be considered when selective inhibitors are unavailable; however, durable remissions are uncommon, and HSCT remains the only reliably curative approach. This is consistent with British Committee for Standards in Haematology recommendations, which advocate HSCT for patients with FGFR1-rearranged clonal eosinophilia or disease refractory to TKI therapy[42] Overall, these observations reinforce the importance of early referral for transplantation, ideally before progression to blast phase. This review has several important limitations. Most available evidence derives from small single-arm trials, retrospective cohorts, or isolated case reports, resulting in very limited sample sizes and inherent risks of selection and publication bias. The absence of randomised controlled trials or other prospective comparative studies precludes both direct and indirect treatment comparisons. In addition, heterogeneity in disease phase, prior treatment exposure, comorbidity burden, and transplant eligibility limits cross-study comparability and introduces the risk of confounding by indication. Variability in endpoint definitions and response criteria, together with relatively short follow-up in several recent studies, restricts the assessment of long-term response durability and safety. Furthermore, the ultra-rare nature of MLN-FGFR1 and the concentration of care within specialized referral centres limit the generalisability of the findings. Data on quality of life, patient-reported outcomes, and cost-effectiveness have not been systematically evaluated. Collectively, these limitations substantially reduce the confidence with which comparative effectiveness and economic implications can be inferred.

Future research should prioritise coordinated multicentre registries and prospective cohorts to strengthen the evidence base for pemigatinib, olverembatinib, and emerging FGFR inhibitors. This is particularly important, given that the very low frequency of MLN-FGFR1 substantially limits the feasibility of adequately powered studies. The development and adoption of harmonised response criteria, particularly those advanced by international working groups, will be essential to enhance comparability and facilitate evidence synthesis in this ultra-rare malignancy. Key unresolved questions include optimal sequencing of pemigatinib with induction chemotherapy, best integration with allogeneic HSCT, and the potential role of combination approaches in blast-phase disease. Further elucidation of resistance mechanisms, including gatekeeper mutations and bypass signalling, will be crucial to guide next-generation therapeutic strategies.

CONCLUSION

Pemigatinib and olverembatinib are the only TKIs with prospective clinical evidence in MLN-FGFR1 and appear to achieve substantially higher and more durable response rates than multi-target inhibitors, with toxicity profiles that are generally consistent with FGFR inhibition but include clinically relevant adverse events. The therapeutic advantage of selective FGFR1 inhibition is most pronounced in chronic-phase disease, whereas long-term disease control continues to depend on timely allogeneic HSCT. Evidence for other TKIs remains limited and inconsistently favourable, with both efficacy and safety data derived mainly from isolated clinical experiences, supporting their secondary role. Continued collaborative research is needed to define optimal treatment sequencing, integration with HSCT, and strategies to minimise toxicity while preventing and overcoming resistance.

AUTHORS'CONTRIBUTIONS
Joanna W. Gomułka designed the study, performed data extraction and synthesis and drafted the manuscript. Joanna W. Gomułka and Konstancja J. Ziegert conducted the literature search, screening, and quality assessment of the studies. Aleksandra A. Mazurkiewicz verified the data. Agnieszka Nadzieja-Kozioł provided critical revision. All authors approved the final manuscript.

CONFLICT OF INTERESTS STATEMENT
Authors are working for MAHTA Sp. z o.o., which performs HTA reports and other services for pharmaceutical companies in Poland.

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SUPPLEMENTARY TABLES

Supplementary Table 1. Search strategy – electronic databases		
#	Query	Results
MEDLINE (via PubMed) [All Fields]		
#1	(("8p11" OR "FGFR1") AND ("myeloproliferative" OR "myeloid") AND ("syndrome" OR "neoplasm"))	268
#2	(("stem cell") AND (leukemia OR leukaemia OR lymphoma) AND ("FGFR1"))	107
#3	#1 OR #2	302
#4	("tki" OR "tyrosine kinase inhibitors" OR "tk inhibitor" OR "protein kinase inhibitors") OR (Targeted AND Therap*)	1,177,018
#5	pemigatinib OR pemazyre OR "incb054828" OR "incb-054828" OR "incb 054828" OR "ibi 375" OR "ibi375" OR "incb 54828" OR "incb54828"	255
#6	midostaurine OR midostaurin OR "cgp 41251" OR "cgp-41251" OR "cgp41251" OR "cgp 41231" OR "cgp41231" OR "pkc412" OR "pkc 412" OR "pkc-412" OR rydapt OR "benzoylstaurosporine"	1,092
#7	ponatinib OR "ap24534" OR "ap 24534" OR "ap-24534" OR iclusig	1,382
#8	#4 OR #5 OR #6 OR #7	1,177,892
#9	#3 AND #8	98
#10	Filters: English, Polish	91
EMBASE		
#1	(('8p11' OR 'fgfr1') AND ('myeloproliferative' OR 'myeloid') AND ('syndrome' OR 'neoplasm')):ti,ab,kw	378
#2	(('stem cell') AND (leukemia OR leukaemia OR lymphoma) AND ('FGFR1')):ti,ab,kw	143
#3	#1 OR #2	432
#4	'tyrosine kinase inhibitors'/syn OR ("tki" OR "tyrosine kinase inhibitors" OR "tk inhibitor" OR "protein kinase inhibitors"):ti,ab,kw	524,209
#5	'molecularly targeted therapy'/syn OR (targeted AND therap*):ti,ab,kw	413,573
#6	(pemigatinib OR pemazyre OR 'incb054828' OR 'incb-054828' OR 'incb 054828' OR 'ibi 375' OR 'ibi375' OR 'incb 54828' OR 'incb54828'):ab,kw,ti,tn	560
#7	(midostaurine OR midostaurin OR 'cgp 41251' OR 'cgp-41251' OR 'cgp41251' OR 'cgp 41231' OR 'cgp41231' OR 'pkc412' OR 'pkc 412' OR 'pkc-412' OR rydapt OR 'benzoylstaurosporine'):ab,kw,ti,tn	3,021
#8	(ponatinib OR 'ap24534' OR 'ap 24534' OR 'ap-24534' OR iclusig):ab,kw,ti,tn	3,477
#9	#4 OR #5 OR #6 OR #7 OR #8	863,403
#10	#3 AND #9	201
#11	#10 AND ([english]/lim OR [polish]/lim)	192
#12	#11 AND ([embase]/lim NOT ([embase]/lim AND [medline]/lim) OR ([medline]/lim NOT ([embase]/lim AND [medline]/lim) NOT ([embase classic]/lim AND [medline]/lim)))	104
Cochrane Central Register of Controlled Trials [All Text]		
#1	(("8p11" OR "FGFR1") AND ("myeloproliferative" OR "myeloid") AND ("syndrome" OR "neoplasm"))	2
#2	(("stem cell") AND (leukemia OR leukaemia OR lymphoma) AND ("FGFR1"))	1
#3	#1 OR #2	2
#4	("tki" OR "tyrosine kinase inhibitors" OR "tk inhibitor" OR "protein kinase inhibitors") OR (Targeted AND Therap*)	89 200
#5	pemigatinib OR pemazyre OR "incb054828" OR "incb-054828" OR "incb 054828" OR "ibi 375" OR "ibi375" OR "incb 54828" OR "incb54828"	25
#6	midostaurine OR midostaurin OR "cgp 41251" OR "cgp-41251" OR "cgp41251" OR "cgp 41231" OR "cgp41231" OR "pkc412" OR "pkc 412" OR "pkc-412" OR rydapt OR "benzoylstaurosporine"	156
#7	ponatinib OR "ap24534" OR "ap 24534" OR "ap-24534" OR iclusig	164
#8	#4 OR #5 OR #6 OR #7	89340
#9	#3 AND #8	2

Search date: 14.11.2025

Supplementary Table 2. Search strategy – congress and trial registers sites			
Ad-hoc searches with web link	Search terms	Results	Included
Congresses			
American Society of Hematology (ASH) 2024-2025 annual meeting ASH 2025 Annual Meeting - Programme 66th ASH Annual Meeting	"FGFR1 rearrangement"	3	2
American Society of Clinical Oncology (ASCO) 2024-2025 annual meeting https://ascopubs.org/search/advanced	"FGFR1 rearrangement"	4	0
European Hematology Association (EHA) 2024-2025 Congress https://library.ehaweb.org/eha/#!*listing=3*browseby=8*sortby=2*media=3*-date=2021-01-01%2000:00:00,2023-12-31%2022:59:59	"FGFR1 rearrangement"	3	2
American Association for Cancer Research (AACR) meeting 2024–2025 https://aacrjournals.org/advanced-search	"FGFR1 rearrangement"	3	0
Society of Hematologic Oncology (SOHO) 2024-2025 Congress https://www.clinical-lymphoma-myeloma-leukemia.com/search/advanced?SeriesKey=clml&ISSN=2152-2650&journalCode=clml	"FGFR1 rearrangement"	5	1
Registries			
Clinical trials https://clinicaltrials.gov/	((("8p11" OR "FGFR1") AND ("myeloproliferative" OR "myeloid") AND ("syndrome" OR "neoplasm"))) OR ((("stem cell") AND (leukemia OR leukaemia OR lymphoma) AND ("FGFR1")))	7	3
International Clinical Trials Registry Platform (ICTRP) https://trialsearch.who.int/Default.aspx	((("8p11" OR "FGFR1") AND ("myeloproliferative" OR "myeloid") AND ("syndrome" OR "neoplasm"))) OR ((("stem cell") AND (leukemia OR leukaemia OR lymphoma) AND ("FGFR1")))	3	3
EU Clinical Trials Register https://www.clinicaltrialsregister.eu/ctr-search/search	((("8p11" OR "FGFR1") AND ("myeloproliferative" OR "myeloid") AND ("syndrome" OR "neoplasm"))) OR ((("stem cell") AND (leukemia OR leukaemia OR lymphoma) AND ("FGFR1")))	2	2

Search date: 14.11.2025

Supplementary Table 3. PICOS criteria		
PICOS	Inclusion criteria	Exclusion criteria
Population	Patients with myeloid/lymphoid neoplasm with FGFR1 rearrangement	Other eosinophilic disorders Healthy subjects In vivo or in vitro or animal studies
Intervention / comparator	Tyrosine kinase inhibitors	Other (e.g. haematopoietic stem cell transplantation, chemotherapy)
Outcome	Efficacy, safety and quality of life	Studies focusing on diagnostic methods, pharmacodynamic and pharmacokinetic parameters
Study type	Systematic reviews with or without meta-analysis	Non-systematic reviews
	Clinical trials (randomized controlled trials or single-arm studies) or observational studies Full-text publications and, where available, only the most recent conference abstracts reporting relevant outcome data for clinical trials and observational studies. Sample size for clinical trials and observational studies: ≥5 participants	Letters, commentaries, editorials, personal opinions Studies without the outcome data Conference abstracts reporting outdated outcome data Sample size for clinical trials and observational studies: <5 participants
	Full text publications for case series or case reports	Case series and case reports available only as conference abstracts (i.e., with no corresponding full-text publication)
	Language: English or Polish	Language other than English or Polish

Supplementary Table 4. Quality Assessment Tool for Before-After (Pre-Post) Studies With No Control Group		
Criteria	FIGHT-203	FIGHT-201
1. Was the study question or objective clearly stated?	Yes	Yes
2. Were eligibility/selection criteria for the study population prespecified and clearly described?	Yes	Yes
3. Were the participants in the study representative of those who would be eligible for the test/service/intervention in the general or clinical population of interest?	Yes	Yes
4. Were all eligible participants that met the prespecified entry criteria enrolled?	Yes	Yes
5. Was the sample size sufficiently large to provide confidence in the findings?	Yes	Yes
6. Was the test/service/intervention clearly described and delivered consistently across the study population?	Yes	Yes
7. Were the outcome measures prespecified, clearly defined, valid, reliable, and assessed consistently across all study participants?	Yes	Yes
8. Were the people assessing the outcomes blinded to the participants' exposures/interventions?	No	No
9. Was the loss to follow-up after baseline 20% or less? Were those lost to follow-up accounted for in the analysis?	Yes	Yes
10. Did the statistical methods examine changes in outcome measures from before to after the intervention? Were statistical tests done that provided p values for the pre-to-post changes?	No	No
11. Were outcome measures of interest taken multiple times before the intervention and multiple times after the intervention (i.e., did they use an interrupted time-series design)?	Yes	Yes
12. If the intervention was conducted at a group level (e.g., a whole hospital, a community, etc.) did the statistical analysis take into account the use of individual-level data to determine effects at the group level?	Yes	Yes

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