AML - 776

Treatment Patterns and Outcomes of Ivosidenib in Patients With mIDH1 AML Previously Treated With Venetoclax-Based Regimens: A Real-World Analysis

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AIM

To assess real-world treatment patterns and outcomes among patients with mutant isocitrate dehydrogenase 1 (mIDH1) acute myeloid leukemia (AML) treated with ivosidenib (IVO)-based regimens following prior venetoclax (VEN) exposure

CONCLUSIONS

- IVO in any treatment line after VEN was associated with a duration of treatment (DOT) of ~6 months and median time to next treatment (TTNT) of >12 months, reflecting meaningful treatment durability and potential tolerability
- Approximately 38% of patients who were initially transfusion dependent (TD) became transfusion independent (TI) during post-VEN IVO treatment, an outcome associated with improved quality of life, reduced number of healthcare visits, and lower treatment burden for patients
- Post-VEN IVO treatment further demonstrated efficacy by bridging a subset (~5%) of patients to stem cell transplant (SCT)
- Clinical benefits were consistent in the subgroup of patients who received second-line (2L) IVO after first-line (1L) VEN, representing a population that is typically older and more comorbid

LIMITATIONS

Limitations inherent to a retrospective claims database, particularly claims data being collected for billing rather than clinical purposes and potentially lacking important clinical details, and small sample sizes should be considered when interpreting these results. Additionally, because *IDH1* mutation status is not captured in claims data, IVO use was considered a proxy for mIDH1 status in this study.

ACKNOWLEDGMENTS

This study was funded by Servier Pharmaceuticals LLC. Medical writing support was provided by Lee Blackburn, MSc, from Amiculum, funded by Servier Pharmaceuticals LLC.

DISCLOSURES

VK reports consultancy relationships with Astellas, Daiichi Sankyo, and Menarini-Stemline; participation in advisory boards for Arog, Omeros, and Servier; receipt of research funding from Eurofins-Viracor and the NIH; and educational honoraria from Blueprint and Bristol Myers Squibb. **TB**, **WL**, **AV**, and **AA** are employees of Servier Pharmaceuticals. **DM** and **UP** are employees of Inizio Ignite Putnam. **AMZ** participated in advisory boards and/or consulted, participated in clinical trial committees for, and/or received honoraria from AbbVie, Akesobio, Agios, Amgen, AstraZeneca/Alexion, Astellas, BioCryst, BioThera, BeOne Medicines, Boehringer Ingelheim, Celgene/BMS, Chiesi/Cornerstone Biopharma, Daiichi Sankyo, Dr Reddy, Epizyme, Faron, Fibrogen/Kyntra Bio, GSK, Glycomimetics, Genentech/Roche, Gilead, Geron, Innocare, Janssen/J&J, Jasper, Karyopharm, Kyowa Kirin, Keros, Kura, Novartis, Notable, Orum, Otsuka, Oncoverity, Pfizer, Puretech/Gallop Oncology, Regeneron, Rigel, Seattle Genetics, Shattuck Labs, Schrodinger, Sumitomo, Syros, Syndax, Servier, Takeda, Treadwell, Taiho, Vincerx, and Zentalis; received clinical trial support (institutional) from AbbVie, Agios, Akesobio, Astex/Taiho, Bristol Myers Squibb/Celgene, Daiichi Sankyo, Faron, Geron, GSK, Genentech/Roche, Kura, Innocare, Novartis, Orum, Oncoverity, Shattuck Labs, Syros, Systimmune, Sillajen, and Takeda; and received travel support from Fibrogen/Kyntra Bio and Kura.

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Presented at: 14th Annual Meeting of the Society of Hematologic Oncology (SOHO 2026), September 9–12, 2026, Houston, Texas, USA

INTRODUCTION

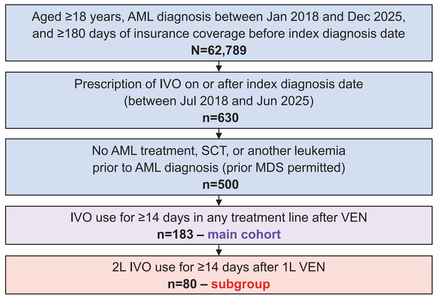
- AML is reported in 4.4 per 100,000 people in the USA, with a 5-year survival rate of 33.4%[1]
- Treatment for patients aged ≥75 years with newly diagnosed mIDH1 AML or ineligible for intensive chemotherapy includes VEN in combination with hypomethylating agents[2]
  - However, additional treatment options in patients aged ≥75 years may be needed as 1L VEN is associated with high relapse rates and poor survival[3]
- IVO is approved as monotherapy or combined with azacitidine for patients with newly diagnosed AML who are aged ≥75 years or who have comorbidities that preclude intensive induction chemotherapy, and for the treatment of adults with relapsed/refractory AML[4]
- Although clinical trials have established the activity of IVO in mIDH1 relapsed/refractory AML,[4] real-world data are limited, particularly in the post-VEN setting

METHODS

Data Source and Study Population

- In this retrospective cohort study, adult patients with AML were identified from the Komodo Research Dataset, which includes longitudinal medical and pharmacy claims for >330 million patients from >150 payers in the USA
- Eligible patients were adults (≥18 years old) with AML who had ≥180 days of continuous insurance coverage before diagnosis; a prescription of IVO on or after index diagnosis; received no prior AML treatment or SCT before AML diagnosis; no history of another leukemia; prior myelodysplastic syndrome permitted; and evidence of IVO use (≥14 days) in any treatment line after VEN-based regimens (**Figure 1**)
  - A subgroup analysis evaluated patients who received 2L IVO after 1L VEN
- Patients were followed up from index AML diagnosis until the earliest of end of insurance coverage or end of data availability in the database

Figure 1: Study Cohort Selection



1L, first line; 2L, second line; AML, acute myeloid leukemia; IVO, ivosidenib; MDS, myelodysplastic syndromes; SCT, stem cell transplant; VEN, venetoclax

Outcomes

- Outcomes evaluated included treatment patterns (DOT and TTNT), the percentage of patients who were TI, defined as ≥8 weeks without transfusion, and the percentage of patients who received SCT

Statistical Analysis

- Baseline characteristics were summarized descriptively
- SCT and TI were assessed using descriptive statistics
- Time-to-event outcomes, including DOT and TTNT, were analyzed using Kaplan–Meier methods

RESULTS

Baseline Characteristics

- A total of 183 patients received IVO in any treatment line after VEN, and 80 patients received 2L IVO after 1L VEN. Median (interquartile range) age was 71 (63–76) and 73 (67–79) years, 51.9% and 63.8% were male, 71.6% and 78.8% received Medicare insurance, 48.6% and 42.5% had a Charlson Comorbidity Index of 0, and 43.7% and 100.0% had one prior treatment, respectively (**Table 1**)
- Median follow-up from treatment start was 8 and 7 months for patients who received IVO in any treatment line after VEN and 2L IVO after 1L VEN, respectively

Table 1: Baseline Patient Characteristics

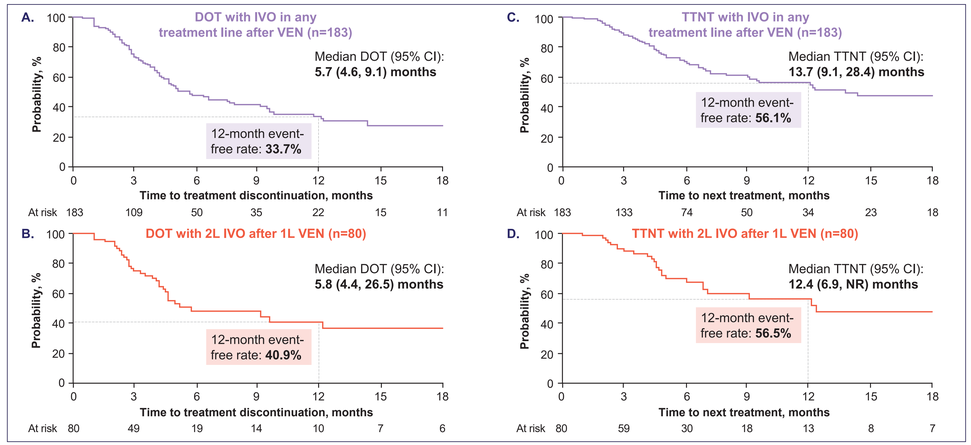
Characteristic	IVO in any treatment line after VEN (n=183)	2L IVO after 1L VEN (n=80)
Sex, n (%)		
Female	84 (45.9)	27 (33.8)
Male	95 (51.9)	51 (63.8)
Unknown	4 (2.2)	2 (2.5)
Age at index diagnosis, median (IQR), years	71 (63–76)	73 (67–79)
Type of insurance, n (%)		
Medicare	131 (71.6)	63 (78.8)
Commercial	42 (23.0)	15 (18.8)
Medicaid	10 (5.5)	2 (2.5)
Region, n (%)		
Midwest	40 (21.9)	16 (20.0)
Northeast	44 (24.0)	16 (20.0)
West	31 (16.9)	15 (18.8)
South	68 (37.2)	33 (41.3)
Available follow-up from index diagnosis, median (IQR), months	19 (12–31)	14 (8–21)
Available follow-up from treatment start, median (IQR), months	8 (4–14)	7 (4–13)
Charlson Comorbidity Index, n (%)		
0	89 (48.6)	34 (42.5)
1–2	48 (26.2)	20 (25.0)
≥3	46 (25.1)	26 (32.5)
Number of prior treatments, n (%)		
1	80 (43.7)	80 (100.0)
2	59 (32.2)	NA
3	26 (14.2)	NA
≥4	18 (9.8)	NA

1L, first line; 2L, second line; IQR, interquartile range; IVO, ivosidenib; NA, not applicable; VEN, venetoclax

Treatment Patterns

- Median DOT was 5.7 months (95% confidence interval [CI] 4.6, 9.1) for patients who received IVO in any treatment line after VEN and 5.8 (4.4, 26.5) for 2L IVO after 1L VEN (**Figure 2A, B**)
- Median TTNT was 13.7 months (95% CI 9.1, 28.4) for patients who received IVO in any treatment line after VEN and 12.4 months (6.9, not reached [NR]) for 2L IVO after 1L VEN (**Figure 2C, D**)
  - An estimated 56.1% remained free from subsequent treatment at 12 months in patients who received IVO in any treatment line after VEN; results were similar in the 2L IVO after 1L VEN subgroup

Figure 2: Treatment Patterns (DOT [A, B] and TTNT [C, D])



1L, first line; 2L, second line; CI, confidence interval; DOT, duration of treatment; IVO, ivosidenib; NR, not reached; TTNT, time to next treatment; VEN, venetoclax

Clinical Outcomes

- SCT occurred in 4.9% (9/183) of patients who received IVO in any treatment line after VEN and in 3.8% (3/80) for 2L IVO after 1L VEN (**Table 2**)
- In patients who were TD prior to initiation of IVO, 37.5% of patients who received IVO in any treatment line after VEN and 51.2% who received 2L IVO after 1L VEN achieved 8-week TI

Table 2: SCT and TI

Clinical outcome	IVO in any treatment line after VEN (n=183)	2L IVO after 1L VEN (n=80)
SCT, n (%)		
Number of patients with transplant (during or post-IVO)	9 (4.9)	3 (3.8)
TI* (RBC/platelet), n (%)		
Number of patients who were TD prior to IVO use	104 (56.8)	43 (53.8)
Patients who were TI by the end of IVO treatment (out of those who were TD prior to IVO use)	39 (37.5)	22 (51.2)

*TI defined as no transfusions over an 8-week period. 1L, first line; 2L, second line; IVO, ivosidenib; RBC, red blood cell; SCT, stem cell transplant; TD, transfusion dependent; TI, transfusion independent; VEN, venetoclax

PLAIN LANGUAGE SUMMARY

Why Was This Study Done?

- People with acute myeloid leukemia (AML) and a change (mutation) in the *IDH1* gene may have cancer that does not respond to treatment (refractory) or comes back after treatment initially worked (relapsed)
- Ivosidenib (IVO) targets the *IDH1* mutation. While IVO has been studied in clinical trials, less is known about how well it works in everyday medical practice, especially for people who have been treated with venetoclax (VEN)

Who Participated in the Study?

- Claims data were used to identify patients with relapsed or refractory AML who had received IVO after VEN in real-world practice in the USA

What Were the Results?

- Patients received IVO for a meaningful duration, and more than half did not start a new treatment until more than 12 months later
- IVO treatment helped some patients stop needing regular blood transfusions for 8 weeks
- These results were consistent in a subset of patients who received IVO as their second treatment after receiving VEN as their first treatment

What Do These Findings Mean for Patients?

- Treatment with IVO after VEN may make cancer treatment easier for patients by lowering the need for blood transfusions and hospital care, while also allowing patients to stay on treatment for longer

REFERENCES

1. National Cancer Institute. U.S. National Institutes of Health, Surveillance, Epidemiology, and End Results Program. Cancer Stat Facts: Leukemia – Acute Myeloid Leukemia (AML). Available at: https://seer.cancer.gov/statfacts/html/amyl.html (accessed August 2026); 2. Shimony S *et al. Am J Hematol* 2025;100:860–91; 3. Abaza Y *et al. Am J Hematol* 2024;99:606–14; 4. Servier Pharmaceuticals LLC. TIBSOVO® (ivosidenib) prescribing information, 2023. Available at: https://www.tibsovo.com/pdf/prescribinginformation.pdf (accessed August 2026).

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