

Tagraxofusp in Adult Blastic Plasmacytoid Dendritic Cell Neoplasm: Clinical Trials and Real-World Outcomes: A Systematic Review

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- Abstract #AML-056



Introduction

Blastic plasmacytoid dendritic cell neoplasm (BPDCN) is a rare, aggressive hematologic malignancy with historically poor outcomes. Tagraxofusp has redefined treatment, yet evidence remains fragmented across clinical trials and real-world studies.

Aim

To systematically evaluate the efficacy and safety of tagraxofusp in adult BPDCN across prospective clinical trials and real-world settings.

Method

- Systematic review of PubMed, Scopus, and Google Scholar, database inception through January 25, 2026
- Adults (≥18 years) with BPDCN treated with tagraxofusp, frontline or relapsed/refractory
- Excluded: narrative reviews, pediatric-only and preclinical studies, abstracts without extractable data
- Outcomes: ORR, CR/CRc, bridging to allogeneic HSCT, and treatment-related toxicity

Conclusions

Tagraxofusp shows robust remission-inducing activity in adult BPDCN across clinical-trial and real-world settings. Long-term outcomes are strongly influenced by successful bridging to HSCT. Prospective comparative and combination studies are needed to optimize sequencing and durability of response.

Results

27 studies • 343 patients

In frontline settings, tagraxofusp achieved an overall response rate (ORR) of 71–90% with complete remission (CR/CRc) rates of 56–72%, establishing it as a highly active induction strategy. Responses in relapsed/refractory disease were lower but remained clinically meaningful. Real-world cohorts showed ORR of 65–90%, reflecting variability in prior therapies and disease burden.

A consistent, clinically significant survival advantage was seen among patients successfully bridged to allogeneic HSCT — highlighting transplantation as a key determinant of long-term outcomes.

Capillary leak syndrome was the hallmark toxicity and primary driver of early treatment-related risk, with variable incidence across settings. Hepatotoxicity and hypoalbuminemia were also frequently observed, reinforcing the need for careful patient selection and monitoring.

27

Studies

343

Patients

Setting	ORR
Frontline	71–90%
Relapsed/refractory	50–67%
Real-world cohorts	65–90%

ORR, overall response rate; CR/CRc, complete remission / complete remission with incomplete count recovery; HSCT, hematopoietic stem-cell transplantation.

Acknowledgements

Publication fees for the related manuscript were supported by a medical grant from Stemline Therapeutics. The funder had no role in study design, data collection, analysis, interpretation, or manuscript preparation. The authors declare no other financial or commercial relationships that could be construed as a conflict of interest.

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References

- Pemmaraju N, Lane AA, Sweet K, et al. Tagraxofusp in blastic plasmacytoid dendritic-cell neoplasm. N Engl J Med. 2019;380:1628-37.
- Pemmaraju N, Sweet KL, Lane AA, et al. Long-term benefits of tagraxofusp for patients with blastic plasmacytoid dendritic cell neoplasm. J Clin Oncol. 2022;40:746-57.
- Frankel AE, Woo JH, Ahn C, et al. Activity of SL-401, a targeted therapy directed to the interleukin-3 receptor, in blastic plasmacytoid dendritic cell neoplasm patients. Blood. 2014;124:385-92.
- Roos-Weil D, Dietrich S, Boumendil A, et al. Stem cell transplantation can provide durable disease control in blastic plasmacytoid dendritic cell neoplasm. Blood. 2013;121:440-6.
- Sandhu M. Evaluation of treatment outcomes in adults with blastic plasmacytoid dendritic cell neoplasm: a systematic review and meta-analysis [abstract]. Blood. 2023;142.

A complete list of the 27 studies included in this systematic review is available upon request.