Population-Based Competing-Risk Analysis of Survival in Blastic Plasmacytoid Dendritic Cell Neoplasm Before and After CD123-Directed Tagraxofusp Therapy Approval

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CONTEXT

Blastic plasmacytoid dendritic cell neoplasm (BPDCN) is a rare, aggressive hematologic malignancy with historically poor prognosis. In December 2018, Tagraxofusp, a CD123-directed fusion toxin, was approved as the first targeted therapy for BPDCN. However, its real-world survival impact remains unquantified in population-level data.

OBJECTIVE

To evaluate differences in BPDCN-specific and overall survival (OS) before and after the approval of Tagraxofusp using U.S. national cancer registry data.

Methodology

We analyzed SEER-17 data (November 2024 submission) to identify patients diagnosed with first-primary BPDCN (ICD-O-3 9727/3) from 2001 to 2022. After excluding cases from 2018 to avoid misclassification, 870 patients were included: 724 diagnosed pre-approval (2001–2017) and 146 post-approval (2019–2022). Outcomes included BPDCN-specific mortality and OS. Kaplan–Meier estimation and multivariable Cox proportional hazards models were used for OS. Fine–Gray competing-risks regression evaluated cancer-specific mortality. Models were adjusted for treatment era, age, sex, race/ethnicity, and summary stage. Sensitivity analyses were conducted in subgroups with ≥24-month potential follow-up and with distant-stage disease.

CONCLUSION

Although all-cause survival remained statistically unchanged, BPDCN-specific mortality declined significantly following Tagraxofusp approval, suggesting early therapeutic benefit. Limitations include short follow-up in the post-approval era and lack of treatment-level data in SEER. These findings highlight the need for extended surveillance, linkage to treatment records, and efforts to ensure equitable access to this novel agent.

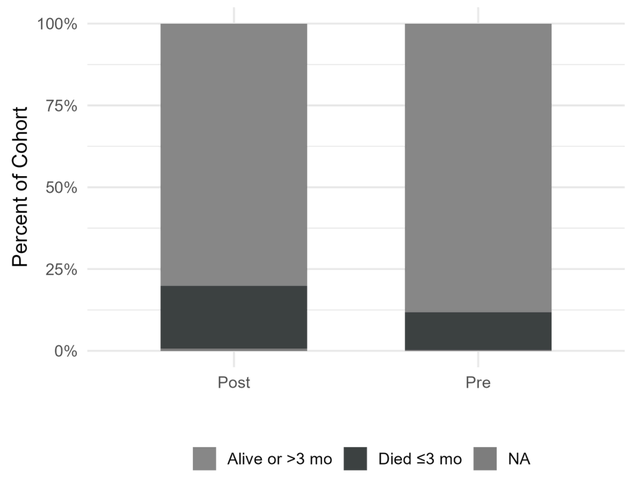
FINANCIAL DISCLOSURE

This author declares no competing financial interests

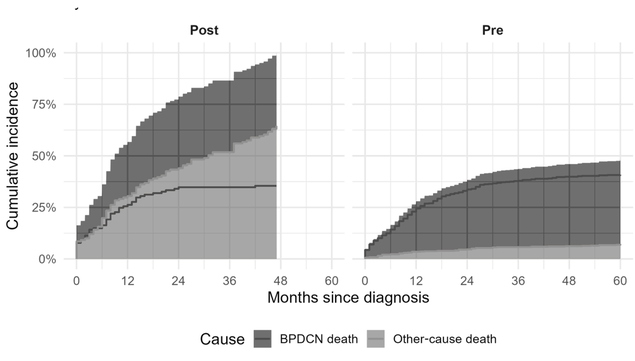
RESULTS

Among 870 patients (median age 63 years; 69% male; 74% White), 457 deaths occurred, 363 (79.4%) of which were BPDCN-specific. Unadjusted 3-month OS was lower post-approval (80.1%) compared to pre-approval (88.5%, $p < 0.01$). Adjusted Cox regression showed no significant difference in OS between eras (HR 0.94; 95% CI 0.70–1.26; $p = 0.70$). However, the subdistribution hazard of BPDCN-specific death was significantly lower post-approval (SHR 0.54; 95% CI 0.38–0.76; $p < 0.001$). Sensitivity analyses confirmed consistent hazard estimates (OS HRs 0.91–1.02; SHR 0.52–0.57).

Early Mortality (<3 Months) by Era



Cummulative Incidence of BPDCN-specific vs other cause death



KEYWORDS

Blastic Plasmacytoid Dendritic Cell Neoplasm, Tagraxofusp, SEER registry, Survival Analysis

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