Abstract: PB2869

Title: THE PATTERNS AND ORGAN TREATMENT RESPONSE OF ERDHEIM-CHESTER DISEASE WITH CARDIAC INVOLVEMENT

Abstract Type: Publication Only

Topic: Myeloproliferative neoplasms - Clinical

Background:

Erdheim-Chester disease (ECD) is a rare histiocytic neoplasm. ECD patients with cardiac involvement display diverse clinical and radiological characteristics, often leading to underdiagnosis. Cohort studies on the patterns and evaluation of ECD cardiac involvement have been scarce.

Aims:

To evaluate the heart response of ECD through continuous follow-up within our large cohort, for which there is a lack of understanding.

Methods:

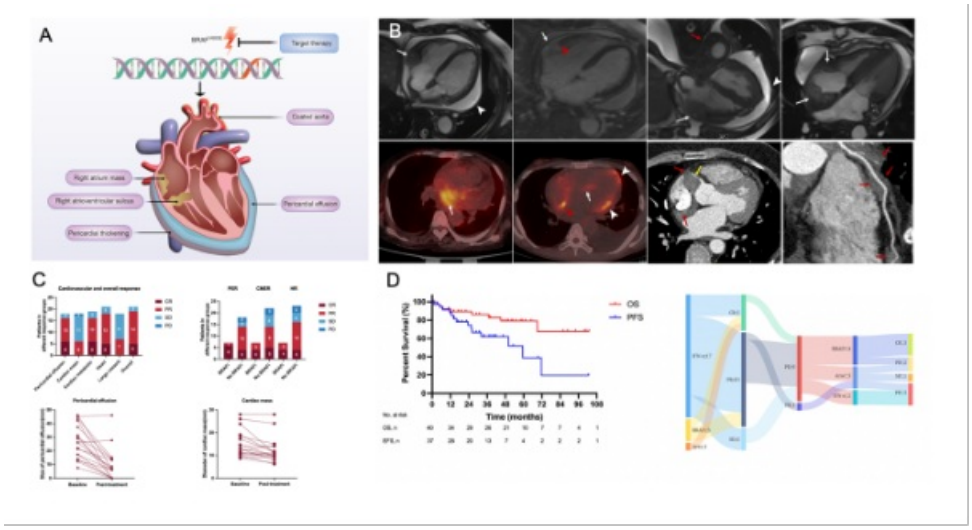
We conducted a retrospective analysis of clinical data from ECD patients with cardiac involvement diagnosed at our center between January 2010 and August 2023. We assessed the heart response by integrating pericardial effusion and metabolic responses.

Results:

A total of 40 patients were included, with a median age of 51.5 years (range: 29–66) and a*BRAFV600E* mutation rate of 56.3%. The most common imaging manifestations observed were pericardial effusion (72.5%), right atrium (70.0%) and right atrioventricular sulcus infiltration (57.5%). Among 21 evaluable patients, 18 (85.7%) achieved a heart response including 5(23.8%) complete response (CR) and 13(61.9%) partial response (PR). The CR rate of pericardial effusion response was 33.3%, while the PR rate was 55.6%. Regarding the cardiac mass response, 33.3% of patients showed PR. For cardiac metabolic response, 31.6% and 52.6% of patients achieved complete and partial metabolic response, respectively. There was a correlation between pericardial effusion response and cardiac metabolic response ($r=0.734$, $p<0.001$). The median follow-up was 50.2 months (range: 1.0-102.8 months). The estimated 5-year overall survival was 78.9%. The median progression-free survival was 59.4 months (95% CI: 26.2-92.7 months). Patients received *BRAF* inhibitors achieved better heart response ($p=0.037$) regardless of treatment lines.

Summary/Conclusion: We pioneered the evaluation of heart response of ECD considering both pericardial effusion and cardiac metabolic response within our cohort, revealing a correlation between these two indicators. *BRAF* inhibitors may improve heart response, regardless of the treatment lines.

Figure legend. (A) Schematic illustrative figure showed cardiac involvement sites of ECD, and BRAFV600E mutation and its target therapy; (B) Cardiac images findings of ECD patients with cardiac involvement; (C) The treatment response of ECD patients with cardiac involvement; (D) Overall survival (OS) of 40 patients and progression-free survival (PFS) of 37 patients received systemic therapy, and heart treatment response of evaluable patients received 2 lines treatment



Keywords: FDG-PET, Histiocytosis, Hematological malignancy