



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

322.DISORDERS OF COAGULATION OR FIBRINOLYSIS: CLINICAL AND EPIDEMIOLOGICAL

Bone Health of Hemophilia A Patients Using EmicizumabTing-Wei Lyu, MBBS¹, Ting Sun¹, Sheng-Chieh Chou²¹Division of Hematology, Department of Internal Medicine, National Taiwan University Hospital, Taipei, Taiwan²Division of Hematology, Department of Internal Medicine, National Taiwan University Hospital, Taipei City, Taiwan

Background:

Hemophilia patients have been observed to exhibit a higher prevalence of osteoporosis compared to age-matched general population. However, the underlying mechanism responsible for this association remains poorly understood. One hypothesis proposes that factor VIII itself may possess additional physiological functions that contribute to bone health. Conversely, alternative viewpoints suggest that overall coagulation function or thrombin generation may play a pivotal role in maintaining optimal bone health. The introduction of emicizumab, a bispecific antibody that mimics the coagulation function of factor VIII but possesses a structurally distinct composition, presents an opportunity to shed light on this matter. Therefore, the primary objective of this study is to explore and compare the bone health status of hemophilia patients, distinguishing between those utilizing emicizumab and those who are emicizumab-naïve.

Method:

From June 2019 to June 2023, at the hemophilia center of National Taiwan University Hospital, additional examinations to assess the patients' bone health were performed with routine annual joint health evaluations for patients with hemophilia A (PwHA) and B (PwHB). This included the use of dual-energy X-ray absorptiometry scan (DEXA) to measure the bone mineral density (BMD) of the lumbar spine, hip, and femur. Furthermore, the serum levels of procollagen type 1 N-terminal propeptide (P1NP) and C-terminal telopeptide (CTX) were measured as reference markers for bone formation and bone resorption, respectively. The Kruskal-Wallis test was utilized to statistically evaluate the observations among the three groups.

Results:

The study population was divided into three distinct groups based on their hemophilia subtype and treatment: PwHA who had been utilizing emicizumab for more than one year (n=8), PwHA individuals receiving regular factor VIII treatment (n=16), and PwHB (n=10). The analysis of the collected data, as presented in the accompanying table, revealed that the three groups exhibited similar characteristics in terms of age, annual bleeding rate, and joint conditions. Moreover, no statistically significant differences were detected in the measurements of bone mineral densities (BMDs) and biomarkers. However, it is worth noting that there appeared to be a potential trend toward higher levels of P1NP in the PwHB group. In the case of PwHA patients using emicizumab, we examined the longitudinal changes in T-score of BMD over time. Notably, three PwHA patients with baseline T-scores below zero demonstrated improvements in their T-scores after one year of emicizumab treatment.

Conclusion:

The results of this study indicate a lack of significant disparities in bone health between individuals utilizing emicizumab and those who are not. Furthermore, our findings suggest that emicizumab usage does not lead to a decline in bone health over the observed period.

Disclosures No relevant conflicts of interest to declare.

Characters of different patient groups

| | Hemophilia A emicizumab user (n=8) | Hemophilia A, emicizumab naïve (n=16) | Hemophilia B (n=10) | P valve |
|------------------|--|---|-------------------------|---------|
| Age | 37.5 (16-53) | 46 (20-70) | 42 (24-66) | 0.804 |
| ABR | 1.5 (0-23) | 2.5 (0-97) | 4 (0-25) | 0.638 |
| BMD Lumbar spine | 0.10 (-2.5-3.4) | -0.25 (-2.9- 1.2) | -0.3 (-2.0-3) | 0.385 |
| BMD femur | -0.7 (-2.2-1.5) | -1.0 (-3.6-1.9) | -1.0 (-2.4-0.5) | 0.978 |
| BMD hip | -1.15 (-2.0-1.5) | -1.0 (-3.2-1.8) | -1.0 (-2.0-0.7) | 0.978 |
| P1NP (ng/mL) | 47.03 (26.78-105.5) | 45.19 (24.05-124.00) | 67.295 (38.88-96.30) | 0.050 |
| CTX (ng/mL) | 0.536 (0.326-0.875) | 0.398 (0.159-0.767) | 0.458 (0.329-0.919) | 0.820 |
| HJHS | 14.50 (2-36) | 17 (1-50) | 10 (2-27) | 0.868 |
| HEAD-US | 22 (5-41) | 24 (1-42) | 14.5 (6-31) | 0.309 |

Data (median, range); ABR: annual bleeding rate; BMD: bone mineral density; P1NP: procollagen type 1 N-terminal propeptide; CTX: C-terminal telopeptide; HJHS: hemophilia joint health score; HEAD-US: hemophilia early arthropathy detection-ultrasound

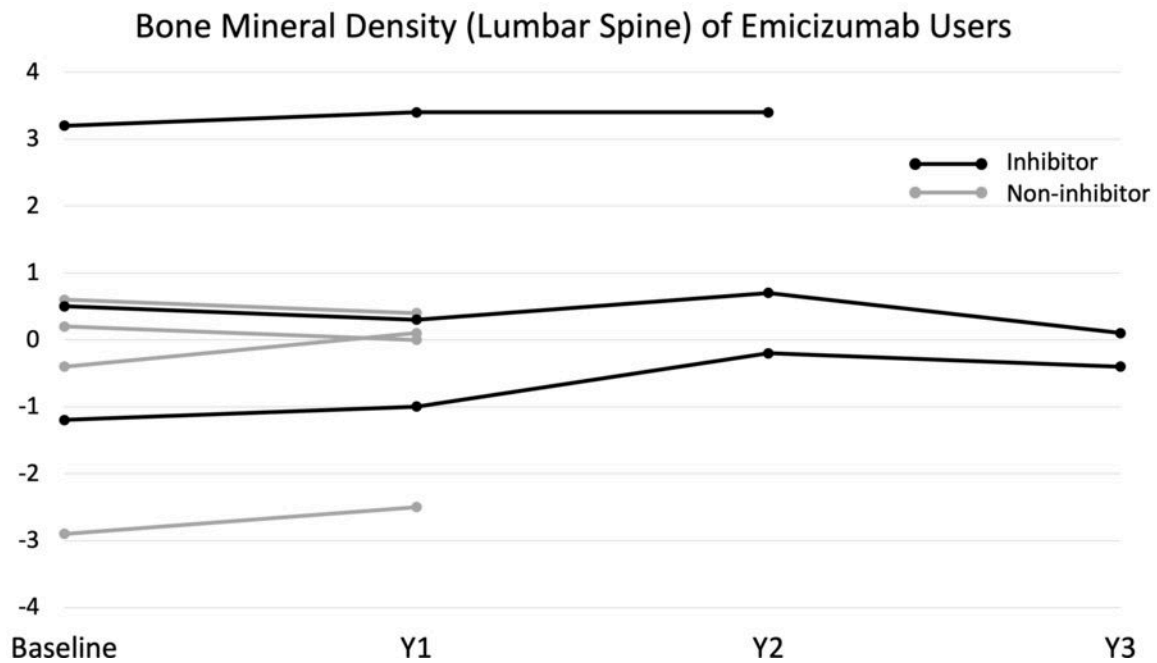


Figure 1

<https://doi.org/10.1182/blood-2023-174200>