Effectiveness and safety of bempedoic acid in routine clinical practice: 1-year follow-up snapshot of the MILOS German cohort

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Background: Low-density lipoprotein cholesterol (LDL-C) has an established role in the development of atherosclerotic cardiovascular disease. The 2019 ESC/EAS dyslipidaemia guidelines recommended more intensive treatment goals, however, many patients do not reach them. Bempedoic acid (BA) is a first-in-class ATP citrate lyase inhibitor that lowers LDL-C levels. There is limited data on the use of BA and BA + ezetimibe (EZE) fixed-dose combination (FDC) in clinical practice.

Purpose: The purpose of this snapshot of 1-year follow-up data from the MILOS study is to evaluate the effectiveness and safety of BA and its FDC in routine clinical practice in Germany.

Methods: MILOS is a European, prospective, observational, non-interventional study in adult patients with primary hypercholesterolaemia or mixed dyslipidaemia. Patients were recruited from 126 sites in Germany between January 2021 and January 2022, and are followed up for 1–2 years after baseline measurements. Here, we summarise 1-year follow-up data from a snapshot taken on January 13th, 2023.

Results: Of 992 patients enrolled in Germany, 714 had either completed their 1-year follow-up or discontinued the study prematurely, and 524 of these 714 patients attended their 1-year follow-up visit. For this snapshot, 714 patients are used when determining cardiovascular (CV) outcomes and safety endpoints, and 524 patients are used when reporting efficacy data. The overall mean (SD) age of the population (n=524) was 64.7 (10.0) years, and 60.7% were male.

Patients treated with BA/BA+EZE FDC had an overall mean LDL-C level of 2.18 mmol/L (84.1 mg/dL) at 1 year compared with 3.20 mmol/L (123.6 mg/dL) pre-treatment, representing a mean relative LDL-C reduction of 27.3%. Overall, 25.8% (135/524) of patients had reached their LDL-C goal after 1 year of treatment compared with 4.0% (21/524) pre-treatment (Figure 1A and 1B). The overall incidence rate of the composite endpoint of major adverse cardiovascular event (4-component MACE) including CV death, non-fatal myocardial infarction, non-fatal stroke, and coronary revascularisation (n=714) was 3.36%. At 1-year follow-up, 19% (136/714) of patients had an adverse drug reaction (ADR) suspected to be related to BA/BA+EZE FDC, and 0.8% (6/714) had serious ADRs, as deemed by the investigator.

Conclusion: This snapshot analysis of 1-year follow-up data suggests that the addition of BA to other lipid-lowering therapies increases the proportion of patients at LDL-C goal by ~6-fold in the observed cohort. The safety and tolerability profile of bempedoic acid are in line with data from randomised controlled trials.
**Figure 1:** Patients at LDL-C goal pre-treatment and at 1-year follow-up in A) the overall group, and B) distribution of patients at LDL-C goal based on investigator-assigned risk at baseline.

**A**

- Overall N=524
- Pre-treatment: 4.01%
- 1-year follow-up: 25.76%

**B**

- Risk category distribution of patients at LDL-C goal (%)
  - Low risk: 0.19%
  - Moderate risk: 0.57%
  - High risk: 1.72%
  - Very high risk: 9.37%
  - Very high risk: 13.74%

LDL-C goals were determined using the updated ESC/EAS 2019 guidelines; this was <70 mg/dL for high-risk patients and <55 mg/dL in very high-risk patients. **ESC**, European Society of Cardiology; **EAS**, European Atherosclerosis Society; **LDL-C**, low-density lipoprotein cholesterol.