ANALYSIS OF THE DISTRIBUTION OF THE ADDED BENEFIT DIMENSIONS IN EARLY BENEFIT ASSESSMENTS DUE TO LABEL EXTENSIONS IN GERMANY

HTA236

Brückel S [1], **Elkashef B** [1] [1] **IGES Institut GmbH**, Nuremberg, Germany

Objectives

In case of a later label extension (LE) of a pharmaceutical a separate early benefit assessment for the new indication shall be conducted. LE are judged to have less high therapeutic value comparing to the first indication. In Germany, pharmaceuticals undergo this assessment as part of the Act on the Reorganisation of the Pharmaceutical Market (AMNOG), which mandates an early benefit assessment to determine the added therapeutic value in comparison to the standard of care. The aim of this research was to analyze the distribution of different dimensions of the additional benefit (AB) in early benefit assessments (EBAs) due to LE, compare it with the distribution of AB in launch procedures and analyze the reasons for an unproven AB in EBAs due to LE.

Methods

The results of all completed EBAs (except reserve antibiotics) by December 2023 due to LE in Germany were analyzed. Qualitative text analysis was used to determine the AB and the reasons for the lack of AB. The main categories depicted the AB and were therefore formed inductively before the analysis of the material. The analysis was performed at the subpopulation level. All information was taken directly from the published G-BA documents using the IGES ARA database.

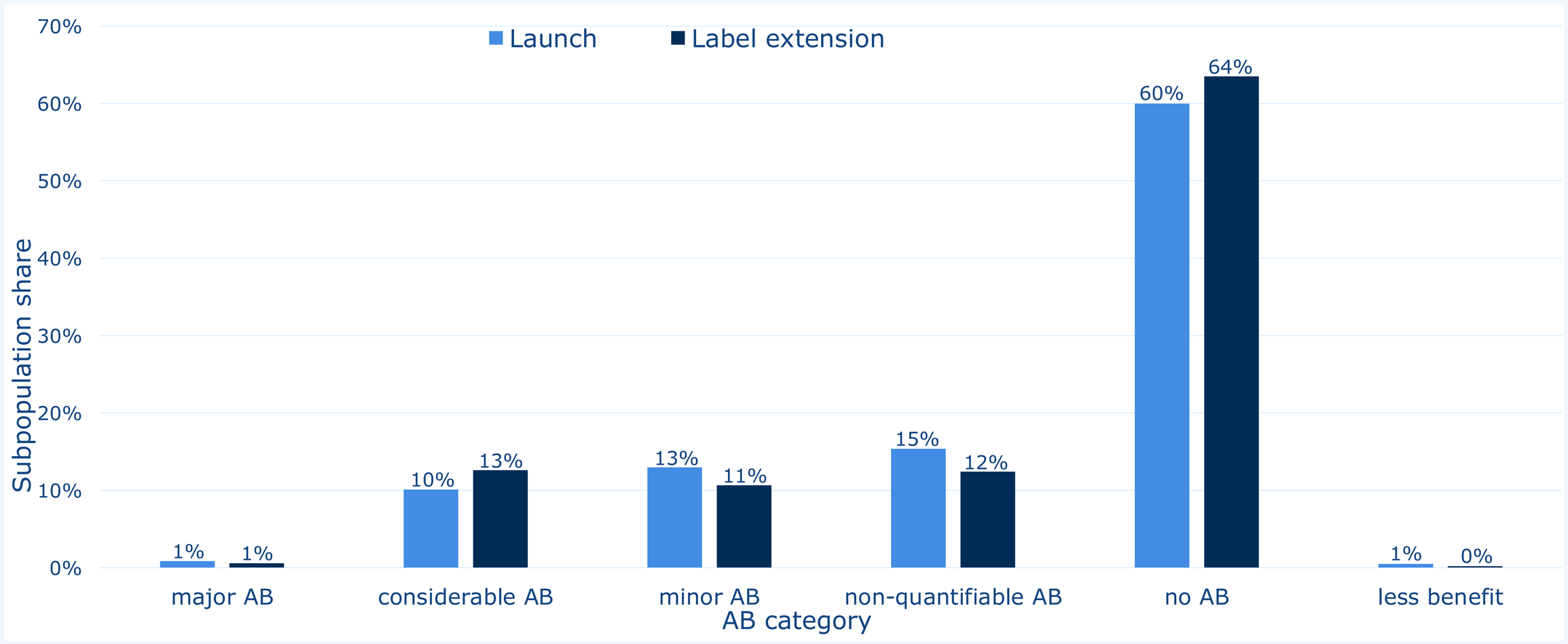
Results

A total of 507 subpopulations for LE were analyzed. A major AB was identified in 3 subpopulations, a considerable AB in 64 subpopulations, a minor AB in 53 subpopulations, and a lesser benefit in 1 subpopulation. In 63 subpopulations, the AB was deemed non-quantifiable, while for 323 subpopulations, no additional benefit could be demonstrated. Figure 1 displays the AB distribution in EBAs for both launches and LEs, showing similar proportions across both, with only minor variations (2-4 percentage points).

Considering LE procedures the most common reason for the absence of an additional benefit was that the submitted study was not suitable for the EBA, accounting for 174 of the 323 subpopulations. Additionally, in 100 subpopulations, no study or relevant data were submitted to support an assessment of additional benefit. In 44 subpopulations, the submitted studies failed to demonstrate a benefit. In 5 subpopulations, the dossiers submitted were either incomplete or entirely missing, preventing a thorough evaluation.

Although randomized controlled trials (RCTs) were frequently submitted, many were deemed inadequate due to a lack of direct comparison with the appropriate comparator therapy.

Figure 1: Subpopulation shares by AB in launch vs. LE EBAs



Conclusions

Despite the criticism that LE generally have lower therapeutic value compared to the initial indications, the distribution of AB among subpopulations in launch procedures and LE was found to be similar. The most common reason for the unproven AB was the unsuitability of submitted evidence for EBA. The challenges facing the pharmaceutical industry in the AMNOG to provide suitable clinical evidence for the EBA become also apparent in the case of label extensions.

References
Gemeinsamer Bundesauschuss: Tragende Gründe zum Beschluss des Gemeinsamen Bundesausschusses über eine Änderung der Arzneimittel-Richtlinie (AM-RL), https://www.g-ba.de/informationen/nutzenbewertung/ (depending on respective EBA)
IGES ARA - AMNOG Resolution Analyzer (https://ara-info.iges.com/Home)