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[C2H2401] Summary of cost-effectiveness evaluation of luspatercept (Reblozyl®)

1. Indications

Myelodysplastic syndrome with anemia

2. Price of the drug

Luspatercept for myelodysplastic syndrome with anemia has been reimbursed since April 2024 at JPY 184,552 for 25 mg, and JPY 551,000 for 75 mg (as of September 2025). The price was calculated based on the cost calculation method, and this product was designated as an H1 cost-effectiveness evaluation item.

3. Scope of cost–effectiveness evaluation

This product is indicated for the treatment of low-risk myelodysplastic syndrome with anemia. The scope of evaluation agreed upon at the first session of the Expert Committee of Cost-Effectiveness Evaluation (ECCEE) is described below:

Population	Low-risk myelodysplastic syndrome with anemia (except patients with deletion 5q)*. (a) Erythropoiesis-stimulating agent (ESA)-naïve patients with ring sideroblast (RS) (b) ESA-naïve patients without RS (c) Patients with RS resistant to, intolerant of, or ineligible for ESA (d) Patients without RS resistant to, intolerant of, or ineligible for ESA *International Prognostic Scoring System Revised very low-, low-, or intermediate-risk

Comparator	(a)(b) Darbepoetin alfa ± best supportive care (BSC) with red blood cell (RBC) transfusions (c)(d) BSC with RBC transfusions

4. Evaluation of additional benefits

The manufacturer cited the Luspatercept clinical trial (COMMANDS) to assess the additional benefits for populations (a) and (b). They concluded that luspatercept had additional benefits over the comparator in population (a), because it produced a superior response regarding RBC transfusion independence. In contrast, they determined that no additional benefits were demonstrated in population (b). For population (c), luspatercept demonstrated additional benefits in another trial (MEDALIST). For population (d), a systematic review (SR) focusing solely on randomized controlled trials (RCT) reported that it was impossible to assess additional benefits because no relevant trials were identified. The academic group conducted SR independently. As the academic group's SR results for populations (a), (b), and (c) were generally consistent with the manufacturer's results, the academic group concluded that the manufacturer's assertion regarding additional benefits in these populations was acceptable. For population (d), the academic group considered the manufacturer’s SR insufficient, expanded it to non-RCTs, and found two single-arm trials (PACE-MDS and MDS-003) that partially included population (d). However, the small sample size and heterogeneity of the study designs make them unsuitable for assessing additional benefits. Furthermore, the academic group examined the clinical trials registered in databases, including ongoing trials, but none were identified. Thus, the academic group concluded that it was not feasible to evaluate the additional benefits of the luspatercept in this population (d).

5. Results of cost-effectiveness analysis

In the cost-effectiveness evaluation, the manufacturer used a Markov model with five health states: "transfusion dependent," "transfusion independent," "high-risk myelodysplastic syndrome," "acute myeloid leukemia," and "death," with quality-adjusted life year (QALY) as the outcome. Regarding population (b), the academic group noted that the manufacturer used treatment continuation rates from the overall population in COMMANDS and applied them separately to each arm. The academic group considered it more appropriate to use data specific to population

(b) and apply pooled continuation rates by combining the two arms. Accordingly, the academic group reanalyzed populations (a) and (b) using the updated price of darbepoetin alfa. The ECCEE accepted the following results.

Population	Comparator	Additional benefit	ICER (JPY/QALY)
(a) ESA-naïve patients with RS	Darbepoetin alfa ± BSC with RBC transfusions	Proven	27,268,507
(b) ESA-naïve patients without RS	Darbepoetin alfa ± BSC with RBC transfusions	No additional benefit	Cost increase
(c) Patients with RS resistant to, intolerant of, or ineligible for ESA	BSC with RBC transfusions	Proven	41,138,889
(d) Patients without RS resistant to, intolerant of, or ineligible for ESA	BSC with RBC transfusions	Impossible to analyze	Impossible to analyze