

To estimate the **Potential Peak Sales** for luspatercept-aamt (REBLOZYL) in the indication of anemia in very low- to intermediate-risk myelodysplastic syndromes with ring sideroblasts (MDS-RS) or myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis (MDS/MPN-RS-T) in the US, EU5 (Germany, France, Italy, Spain, UK), China, and Japan, as well as the **\$ value of 1% share of treated patients** in these geographies, we need to follow a structured approach. Since specific data (e.g., exact patient numbers, pricing, or market penetration rates) are not provided, I will outline the methodology and use reasonable assumptions based on publicly available information and industry standards.

Step 1: Define the Target Patient Population

The indication is for adult patients with very low- to intermediate-risk MDS-RS or MDS/MPN-RS-T who have anemia failing an erythropoiesis-stimulating agent (ESA) and require ≥ 2 RBC units over 8 weeks. Myelodysplastic syndromes (MDS) are rare hematologic disorders, and the specific subset with ring sideroblasts (RS) and the defined treatment criteria further narrows the population.

- **US:** Estimated MDS prevalence is ~60,000-170,000 patients, with an incidence of ~10,000-20,000 new cases/year. Very low- to intermediate-risk MDS-RS accounts for ~15-20% of MDS cases. Of these, ~30-40% may fail ESA and require frequent transfusions. Assuming a midpoint, the target population might be ~3,000-5,000 patients.

- **EU5:** Combined population is roughly similar to the US in terms of MDS prevalence adjusted for population size (~300 million vs. 330 million in the US). Target population might be ~3,000-5,000 patients.

- **China:** With a population of ~1.4 billion, MDS prevalence is less well-documented, but incidence may be lower due to younger demographics. Assuming a conservative estimate, the target population might be ~5,000-8,000 patients.

- **Japan:** With a population of ~125 million and a high proportion of elderly individuals, MDS incidence is significant. Target population might be ~1,500-2,500 patients.

Total Target Population (approximate):

- US: ~4,000 patients
- EU5: ~4,000 patients
- China: ~6,500 patients
- Japan: ~2,000 patients
- **Total:** ~16,500 patients

Step 2: Estimate Treated Patient Share (20%-30%)

Assuming a 20%-30% share of treated patients as given in the query:

- At 20% share: ~3,300 patients treated globally.
- At 30% share: ~4,950 patients treated globally.

Breakdown by Geography (at 20%-30% share):

- US: 800-1,200 patients

- EU5: 800-1,200 patients
- China: 1,300-1,950 patients
- Japan: 400-600 patients

Step 3: Estimate Annual Cost of Therapy

Luspatercept-aamt (REBLOZYL) is a biologic drug administered via subcutaneous injection every 3 weeks. Pricing varies by region:

- **US:** The annual cost is estimated at ~\$100,000-125,000 per patient based on reported figures and similar biologics (e.g., wholesale acquisition cost and dosing schedules).
- **EU5:** Pricing is typically 30-50% lower than the US due to healthcare system negotiations. Assume ~\$60,000-80,000 per patient annually.
- **China:** Pricing is often significantly lower due to market access challenges and local pricing policies. Assume ~\$30,000-40,000 per patient annually.
- **Japan:** Pricing is closer to EU levels, ~\$60,000-80,000 per patient annually.

Midpoint Annual Cost Assumptions:

- US: \$112,500
- EU5: \$70,000
- China: \$35,000
- Japan: \$70,000

Step 4: Calculate Potential Peak Sales (20%-30% Share)

Peak sales are calculated as (number of treated patients) × (annual cost per patient) for each geography.

At 20% Share:

- **US:** 800 patients × \$112,500 = **\$90 million**
- **EU5:** 800 patients × \$70,000 = **\$56 million**
- **China:** 1,300 patients × \$35,000 = **\$45.5 million**
- **Japan:** 400 patients × \$70,000 = **\$28 million**
- **Total Peak Sales (20% share):** \$90M + \$56M + \$45.5M + \$28M = **\$219.5 million**

At 30% Share:

- **US:** 1,200 patients × \$112,500 = **\$135 million**
- **EU5:** 1,200 patients × \$70,000 = **\$84 million**
- **China:** 1,950 patients × \$35,000 = **\$68.25 million**
- **Japan:** 600 patients × \$70,000 = **\$42 million**

- **Total Peak Sales (30% share):** \$135M + \$84M + \$68.25M + \$42M = **\$329.25 million**

Potential Peak Sales Range: \$219.5 million to \$329.25 million annually across the specified geographies for this indication.

Step 5: Calculate \$ Value of 1% Share of Treated Patients

1% share of the total target population (~16,500 patients) is 165 patients. Using the same annual cost per patient as above:

- **US:** 1% = 40 patients × \$112,500 = **\$4.5 million**

- **EU5:** 1% = 40 patients × \$70,000 = **\$2.8 million**

- **China:** 1% = 65 patients × \$35,000 = **\$2.275 million**

- **Japan:** 1% = 20 patients × \$70,000 = **\$1.4 million**

- **Total \$ Value of 1% Share:** \$4.5M + \$2.8M + \$2.275M + \$1.4M = **\$10.975 million**

Final Answer:

1. **Potential Peak Sales for luspatercept-aamt** in the specified indication (20%-30% share of treated patients):

- **Range: \$219.5 million to \$329.25 million** annually across the US, EU5, China, and Japan.

- Breakdown by geography:

- US: \$90M - \$135M

- EU5: \$56M - \$84M

- China: \$45.5M - \$68.25M

- Japan: \$28M - \$42M

2. **\$ Value of 1% Share of Treated Patients:**

- **Total: \$10.975 million** annually across all geographies.

- Breakdown by geography:

- US: \$4.5M

- EU5: \$2.8M

- China: \$2.275M

- Japan: \$1.4M

Notes and Caveats:

- These estimates are based on assumptions for patient population, pricing, and market penetration. Actual figures may vary due to real-world data on MDS-RS prevalence, reimbursement policies, competition, and uptake rates.

- Peak sales assume steady-state market penetration, which may take several years post-launch to achieve.
- Pricing in China and other markets may be subject to significant variation due to local policies and access programs.
- Additional indications for luspatercept-aamt (e.g., beta-thalassemia) are not included in this analysis but could impact overall sales.