

To estimate the **Potential Peak Sales** for imetelstat (Rytelo) in the indication of low- to intermediate-1 risk myelodysplastic syndromes (MDS) with transfusion-dependent anemia in the US, EU5 (France, Germany, Italy, Spain, UK), China, and Japan, as well as the **\$ value of a 1% share of treated patients** in these geographies, we need to follow a structured approach. This involves estimating the eligible patient population, the share of treated patients, pricing assumptions, and market penetration.

Since specific data on pricing, exact patient numbers, and market penetration for imetelstat in this indication are not publicly available in real-time as of now, I will use reasonable assumptions based on industry standards, epidemiology data for MDS, and typical pricing for rare disease or oncology drugs. I will also assume a 20-30% share of treated patients as per your query.

Step 1: Define the Target Patient Population

Imetelstat is approved for adults with low- to intermediate-1 risk MDS with transfusion-dependent anemia who are ineligible for or have not responded to erythropoiesis-stimulating agents (ESAs). This is a subset of MDS patients.

Epidemiology of MDS:

- MDS incidence is approximately 4-5 per 100,000 people annually in Western countries, with higher rates in older populations.
- Low- to intermediate-1 risk MDS accounts for ~60-70% of MDS cases.
- Of these, ~30-40% are transfusion-dependent.
- Of transfusion-dependent patients, a significant portion (estimated 50-60%) may be ineligible for or fail ESAs.

Estimated Eligible Patient Population:

Using rough estimates based on population size and MDS epidemiology:

- **US:** Population ~330M, MDS prevalence ~60,000-170,000 (active cases). Low- to intermediate-1 risk ~40,000-100,000. Transfusion-dependent subset ~12,000-40,000. ESA-ineligible/failed ~6,000-24,000.
- **EU5:** Population ~320M, similar MDS prevalence, eligible population ~5,000-20,000.
- **Japan:** Population ~125M, eligible population ~2,000-8,000.
- **China:** Population ~1.4B, but lower diagnosis rates and access to treatment. Eligible population ~10,000-40,000 (conservative due to healthcare access).

For simplicity, let's use midpoint estimates for eligible patients:

- US: 15,000
- EU5: 12,500
- Japan: 5,000
- China: 25,000
- **Total eligible patients across geographies:** ~57,500

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

Assuming imetelstat captures 20-30% of the eligible patient population as treated patients:

- At 20% share: $57,500 \times 0.20 = \mathbf{11,500 \text{ treated patients}}$
- At 30% share: $57,500 \times 0.30 = \mathbf{17,250 \text{ treated patients}}$

Breakdown by Geography (at 25% average share for simplicity in distribution):

- US: $15,000 \times 0.25 = 3,750 \text{ treated patients}$
- EU5: $12,500 \times 0.25 = 3,125 \text{ treated patients}$
- Japan: $5,000 \times 0.25 = 1,250 \text{ treated patients}$
- China: $25,000 \times 0.25 = 6,250 \text{ treated patients}$
- **Total treated patients (at 25% share): 14,375**

Step 3: Pricing Assumptions

Imetelstat is a novel therapy for a rare indication, so pricing will likely be high, similar to other orphan drugs or oncology treatments. Pricing varies by region due to healthcare systems and purchasing power:

- **US:** Assume annual cost per patient ~\$150,000–\$200,000 (midpoint \$175,000). This is typical for rare disease drugs.
- **EU5:** Assume ~60-70% of US pricing due to negotiations and health systems, ~\$105,000–\$122,500 (midpoint \$115,000).
- **Japan:** Assume ~80% of US pricing, ~\$140,000.
- **China:** Assume significantly lower pricing due to market access and affordability, ~\$30,000–\$50,000 (midpoint \$40,000).

Step 4: Calculate Potential Peak Sales

Peak sales are calculated as: **Number of treated patients × Annual cost per patient.**

At 25% Share of Treated Patients (midpoint estimate for simplicity):

- **US:** $3,750 \text{ patients} \times \$175,000 = \mathbf{\$656.25 \text{ million}}$
- **EU5:** $3,125 \text{ patients} \times \$115,000 = \mathbf{\$359.38 \text{ million}}$
- **Japan:** $1,250 \text{ patients} \times \$140,000 = \mathbf{\$175.00 \text{ million}}$

- **China:** 6,250 patients × \$40,000 = **\$250.00 million**
- **Total Peak Sales (at 25% share):** \$656.25M + \$359.38M + \$175.00M + \$250.00M = **~\$1.44 billion**

Range of Peak Sales (20-30% Share):

- At 20% share (11,500 treated patients):
- US: 3,000 × \$175,000 = \$525M
- EU5: 2,500 × \$115,000 = \$287.5M
- Japan: 1,000 × \$140,000 = \$140M
- China: 5,000 × \$40,000 = \$200M
- **Total: ~\$1.15 billion**
- At 30% share (17,250 treated patients):
- US: 4,500 × \$175,000 = \$787.5M
- EU5: 3,750 × \$115,000 = \$431.25M
- Japan: 1,500 × \$140,000 = \$210M
- China: 7,500 × \$40,000 = \$300M
- **Total: ~\$1.73 billion**

Potential Peak Sales Range: \$1.15 billion to \$1.73 billion annually across the US, EU5, Japan, and China, with a midpoint of ~\$1.44 billion at 25% share.

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

A 1% share of the eligible patient population (57,500) is 575 treated patients. Using the same pricing assumptions:

- **US:** 1% of 15,000 = 150 patients × \$175,000 = **\$26.25 million**
- **EU5:** 1% of 12,500 = 125 patients × \$115,000 = **\$14.38 million**
- **Japan:** 1% of 5,000 = 50 patients × \$140,000 = **\$7.00 million**
- **China:** 1% of 25,000 = 250 patients × \$40,000 = **\$10.00 million**
- **Total \$ Value of 1% Share:** \$26.25M + \$14.38M + \$7.00M + \$10.00M = **~\$57.63 million**

Final Answer:

1. **Potential Peak Sales for Imetelstat** in the US, EU5, China, and Japan for low- to intermediate-1 risk MDS with transfusion-dependent anemia (assuming 20-30% share of treated patients):

- Range: **\$1.15 billion to \$1.73 billion annually**
- Midpoint (at 25% share): **~\$1.44 billion annually**

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

- **~\$57.63 million annually**

Caveats: These estimates are based on assumptions for patient population, market share, and pricing. Real-world figures may vary based on actual drug pricing, market access, competition (e.g., other MDS therapies), reimbursement policies, and penetration rates in different regions, especially in China where healthcare access and affordability are significant factors. For more precise estimates, primary market research or data from Geron Corporation's financial projections would be needed.