

To estimate the **Potential Peak Sales** for treosulfan in the specified indication (as a preparative regimen for allogeneic hematopoietic stem cell transplantation (alloHSCT) in patients with acute myeloid leukemia (AML) or myelodysplastic syndrome (MDS)) in the US, EU5 (Germany, France, 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. Since specific data such as exact patient numbers, pricing, or market penetration rates are not provided, I will outline the methodology and make reasonable assumptions based on publicly available data and industry trends.

Step 1: Define the Target Patient Population

Treosulfan is approved for AML and MDS patients undergoing alloHSCT. We need to estimate the number of eligible patients in each geography.

- **AML and MDS Incidence:** AML and MDS are rare hematological malignancies. According to various sources:
- **US:** Approximately 20,000 new AML cases and 10,000 MDS cases annually. Of these, about 20-30% may be eligible for alloHSCT (considering age, disease stage, and comorbidities), leading to roughly 6,000-9,000 eligible patients.
- **EU5:** Combined population is roughly similar to the US (~330 million). Incidence rates are comparable, so approximately 5,000-8,000 eligible patients for alloHSCT.
- **Japan:** Population ~125 million. AML/MDS incidence adjusted for population suggests ~2,000-3,000 eligible patients.
- **China:** Population ~1.4 billion. Higher incidence due to population size, but lower access to advanced treatments like alloHSCT. Estimated 10,000-15,000 eligible patients, though actual treated numbers may be lower due to healthcare access constraints.

- Total Eligible Patients for alloHSCT (rough estimate):

- US: ~7,500
- EU5: ~6,500
- China: ~12,000 (assuming lower penetration)
- Japan: ~2,500
- **Total:** ~28,500 patients annually across all geographies.

- **Share of Treated Patients:** Assuming 20-30% of eligible patients are treated with treosulfan (as per the query), we estimate:

- 20% share: ~5,700 patients treated.
- 30% share: ~8,550 patients treated.

Step 2: Estimate Drug Pricing

Treosulfan pricing varies by region due to differences in healthcare systems and purchasing power. Based on industry benchmarks for oncology drugs and conditioning regimens:

- **US:** ~\$50,000 per treatment course (high due to premium pricing).

- **EU5:** ~\$30,000 per course (lower due to negotiated pricing).
- **Japan:** ~\$35,000 per course (similar to EU but with slight premium).
- **China:** ~\$15,000 per course (lower due to cost constraints and generics competition).

Step 3: Calculate Potential Peak Sales

Peak sales are calculated by multiplying the number of treated patients by the price per treatment course in each geography.

At 20% Share of Treated Patients:

- **US:** 7,500 patients * 20% = 1,500 patients * \$50,000 = **\$75 million**.
- **EU5:** 6,500 patients * 20% = 1,300 patients * \$30,000 = **\$39 million**.
- **China:** 12,000 patients * 20% = 2,400 patients * \$15,000 = **\$36 million**.
- **Japan:** 2,500 patients * 20% = 500 patients * \$35,000 = **\$17.5 million**.
- **Total Peak Sales (20%):** \$75M + \$39M + \$36M + \$17.5M = **\$167.5 million**.

At 30% Share of Treated Patients:

- **US:** 7,500 patients * 30% = 2,250 patients * \$50,000 = **\$112.5 million**.
- **EU5:** 6,500 patients * 30% = 1,950 patients * \$30,000 = **\$58.5 million**.
- **China:** 12,000 patients * 30% = 3,600 patients * \$15,000 = **\$54 million**.
- **Japan:** 2,500 patients * 30% = 750 patients * \$35,000 = **\$26.25 million**.
- **Total Peak Sales (30%):** \$112.5M + \$58.5M + \$54M + \$26.25M = **\$251.25 million**.

Potential Peak Sales Range: \$167.5 million to \$251.25 million annually across the specified geographies.

Step 4: Calculate \$ Value of 1% Share of Treated Patients

A 1% share of treated patients corresponds to 1% of the eligible patient population being treated with treosulfan.

- **US:** 7,500 patients * 1% = 75 patients * \$50,000 = **\$3.75 million**.
- **EU5:** 6,500 patients * 1% = 65 patients * \$30,000 = **\$1.95 million**.
- **China:** 12,000 patients * 1% = 120 patients * \$15,000 = **\$1.8 million**.
- **Japan:** 2,500 patients * 1% = 25 patients * \$35,000 = **\$0.875 million**.
- **Total \$ Value of 1% Share:** \$3.75M + \$1.95M + \$1.8M + \$0.875M = **\$8.375 million**.

Final Answer:

- **Potential Peak Sales for Treosulfan** (20-30% share of treated patients):

- **Range: \$167.5 million to \$251.25 million annually** across the US, EU5, China, and Japan.
- **\$ Value of 1% Share of Treated Patients:**
- **Total: \$8.375 million annually** across the specified geographies.

Notes and Caveats:

1. These estimates are based on assumptions about patient populations, pricing, and market penetration. Real-world data may differ due to factors like competition (e.g., other conditioning regimens like busulfan), reimbursement policies, and regional healthcare access.
2. Peak sales may take several years to achieve post-launch due to gradual market adoption.
3. China's numbers are particularly uncertain due to variability in healthcare access and potential pricing pressures. Adjustments may be needed based on local market dynamics.
4. If you have specific data on patient numbers, pricing, or market share, these estimates can be refined further.