

To estimate the **Potential Peak Sales** for axatilimab-csfr (Niktimvo) in the indication of chronic graft-versus-host disease (cGVHD) 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 based on available data and reasonable assumptions. Since specific pricing, patient population, and market penetration data for axatilimab-csfr are not publicly available in full detail at this time, I will outline the methodology and use realistic estimates for key parameters.

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## **Step 1: Define the Target Patient Population**

cGVHD is a complication of allogeneic hematopoietic stem cell transplantation (HSCT). It typically affects patients after failure of at least two prior lines of systemic therapy, as per the FDA approval for axatilimab-csfr.

### #### Incidence and Prevalence of cGVHD

- **US:** Approximately 14,000–20,000 HSCT procedures are performed annually. About 30–50% of these patients develop cGVHD, with a subset failing two prior therapies. Estimated prevalent cGVHD patients failing two lines of therapy: ~5,000–7,000.
- **EU5:** HSCT volume is similar to the US, with ~15,000–20,000 procedures annually. Prevalence of cGVHD failing two lines: ~5,000–7,000.
- **Japan:** HSCT volume is lower, ~5,000–7,000 annually. Prevalence of cGVHD failing two lines: ~1,500–2,000.
- **China:** HSCT volume is growing, estimated at ~10,000–15,000 annually. Prevalence of cGVHD failing two lines: ~3,000–5,000.

Total target patient population across these geographies: ~14,500–21,000 patients.

### #### Treated Patients

Assuming 80–90% of eligible patients receive treatment (due to access, cost, or clinical decisions), the treated population is ~11,600–18,900 patients.

### #### Market Share Assumption

As per the query, axatilimab-csfr is assumed to capture **20–30%** of treated patients.

- Treated patients captured by axatilimab-csfr: **2,320–5,670** (20–30% of 11,600–18,900).

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## **Step 2: Estimate Annual Cost of Therapy**

The pricing for axatilimab-csfr is not publicly available, but we can benchmark it against other biologics for rare diseases or cGVHD treatments like ibrutinib (Imbruvica) or belumosudil (Rezurock), which cost ~\$150,000–\$200,000 per patient per year in the US. Assuming axatilimab-csfr is priced similarly:

- **US:** \$175,000 per patient per year.

- **EU5:** Pricing is typically 60–70% of US prices due to negotiations and healthcare systems: ~\$105,000–\$122,500 per patient per year (average \$115,000).
- **Japan:** Pricing is similar to EU5, ~\$115,000 per patient per year.
- **China:** Pricing is significantly lower due to market dynamics and access programs, ~\$50,000 per patient per year.

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### **Step 3: Calculate Potential Peak Sales**

Peak sales are calculated as:

**Peak Sales = Number of Treated Patients Captured × Annual Cost of Therapy**

#### US

- Treated patients captured (20–30%): ~1,000–2,100 (based on 5,000–7,000 total treated patients).
- Annual cost: \$175,000.
- Peak sales: **\$175M–\$367.5M.**

#### EU5

- Treated patients captured (20–30%): ~1,000–2,100.
- Annual cost: \$115,000.
- Peak sales: **\$115M–\$241.5M.**

#### Japan

- Treated patients captured (20–30%): ~300–600.
- Annual cost: \$115,000.
- Peak sales: **\$34.5M–\$69M.**

#### China

- Treated patients captured (20–30%): ~600–1,500.
- Annual cost: \$50,000.
- Peak sales: **\$30M–\$75M.**

#### Total Peak Sales Across Geographies

- Total peak sales (20% share): **\$354.5M** (\$175M US + \$115M EU5 + \$34.5M Japan + \$30M China).
- Total peak sales (30% share): **\$753M** (\$367.5M US + \$241.5M EU5 + \$69M Japan + \$75M China).
- **Range for Potential Peak Sales: \$354.5M–\$753M.**

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

1% share of treated patients corresponds to 1% of the total treated population (11,600–18,900), i.e., ~116–189 patients.

### **#### US**

- 1% of treated patients: ~50–70.
- Annual cost: \$175,000.
- Value of 1% share: **\$8.75M–\$12.25M.**

### **#### EU5**

- 1% of treated patients: ~50–70.
- Annual cost: \$115,000.
- Value of 1% share: **\$5.75M–\$8.05M.**

### **#### Japan**

- 1% of treated patients: ~15–20.
- Annual cost: \$115,000.
- Value of 1% share: **\$1.73M–\$2.3M.**

### **#### China**

- 1% of treated patients: ~30–50.
- Annual cost: \$50,000.
- Value of 1% share: **\$1.5M–\$2.5M.**

### **#### Total \$ Value of 1% Share Across Geographies**

- Total value (lower end): **\$17.73M** (\$8.75M US + \$5.75M EU5 + \$1.73M Japan + \$1.5M China).
- Total value (upper end): **\$25.1M** (\$12.25M US + \$8.05M EU5 + \$2.3M Japan + \$2.5M China).
- **Range for \$ Value of 1% Share: \$17.73M–\$25.1M.**

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## **Final Answer**

- **Potential Peak Sales for axatilimab-csfr (20–30% share of treated patients): \$354.5M–\$753M** across the US, EU5, China, and Japan.
- **\$ Value of 1% Share of Treated Patients: \$17.73M–\$25.1M** across the same geographies.

## **Notes and Caveats**

1. These estimates are based on assumptions about patient population, market penetration, and pricing. Actual figures may vary based on real-world data, competition (e.g., other cGVHD therapies like Jakafi, Imbruvica, Rezurock), reimbursement policies, and market access.
2. Peak sales typically occur 5–7 years post-launch, assuming no major disruptions (e.g., patent expiry, biosimilars).
3. China's market potential may be underestimated due to growing HSCT volumes and improving access to expensive therapies, though pricing remains a significant barrier.