

To estimate the **Potential Peak Sales** for ruxolitinib (Jakafi) 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. Since specific data such as patient population, pricing, and penetration rates are not provided, I will outline the methodology and make reasonable assumptions based on publicly available information and market trends for rare disease drugs. The final numbers are illustrative and should be validated with real-world data.

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## **Step 1: Define the Target Population for cGVHD**

cGVHD is a complication following allogeneic hematopoietic stem cell transplantation (HSCT). It typically affects 30-70% of HSCT recipients, with varying incidence and prevalence across regions due to differences in transplant rates, patient demographics, and healthcare access.

#### Estimated cGVHD Patient Population:

- **US:** Approximately 14,000-20,000 new HSCTs are performed annually. Assuming 50% develop cGVHD, and focusing on patients failing 1-2 lines of therapy (around 30-40% of cGVHD cases), the eligible population is ~2,100-3,200 patients annually.
- **EU5:** HSCT rates are similar to the US on a per capita basis. With a population roughly 1.5x that of the US, the eligible cGVHD population is ~3,150-4,800 patients annually.
- **Japan:** Japan has a lower HSCT rate (around 5,000-6,000 annually). Assuming similar cGVHD incidence and treatment failure rates, the eligible population is ~750-1,200 patients annually.
- **China:** China has a rapidly growing HSCT market, with estimates of 10,000-15,000 HSCTs annually. However, access to advanced therapies like ruxolitinib may be limited. Assuming a smaller proportion of eligible patients (20-30% of cGVHD cases failing prior therapy), the population is ~1,500-3,000 patients annually.

**Total Eligible Population (Annual Incidence):**

- US: ~2,100-3,200
- EU5: ~3,150-4,800
- Japan: ~750-1,200
- China: ~1,500-3,000
- **Total:** ~7,500-12,200 patients annually

Since cGVHD is a chronic condition, the **prevalent population** (patients living with the disease and eligible for treatment) may be 2-3x the annual incidence, i.e., ~15,000-36,600 patients across these geographies.

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## **Step 2: Estimate Market Penetration (20%-30% Share of Treated Patients)**

Assuming ruxolitinib captures **20%-30% of treated patients** in this indication (post-failure of 1-2 lines of therapy), we calculate the treated patient share:

**- Total Treated Patients at Peak Penetration:**

- 20% share: 3,000-7,320 patients
- 30% share: 4,500-10,980 patients

This assumes that not all eligible patients are treated due to access, cost, or alternative therapies, and penetration varies by region (higher in the US and EU5, lower in China).

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### **Step 3: Estimate Annual Treatment Cost per Patient**

Ruxolitinib is a high-cost specialty drug. Based on its pricing for other indications (e.g., myelofibrosis, polycythemia vera), the annual cost per patient is approximately:

- **US:** ~\$150,000-\$180,000 per year (based on list price, though discounts and rebates apply)
- **EU5:** ~\$80,000-\$120,000 per year (lower due to pricing negotiations and healthcare systems)
- **Japan:** ~\$100,000-\$130,000 per year (similar to EU5 but with regional pricing differences)
- **China:** ~\$40,000-\$60,000 per year (significantly lower due to pricing controls and generics/biosimilars)

For simplicity, we use a blended average cost across regions, weighted by patient distribution:

- Weighted Average Cost: ~\$100,000 per patient per year (higher in US/EU5/Japan, lower in China)

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### **Step 4: Calculate Potential Peak Sales**

Peak sales are calculated as:

**Peak Sales = Number of Treated Patients x Annual Cost per Patient**

- **At 20% Market Share** (3,000-7,320 patients):
  - Peak Sales = 3,000 x \$100,000 to 7,320 x \$100,000 = **\$300M to \$732M annually**
- **At 30% Market Share** (4,500-10,980 patients):
  - Peak Sales = 4,500 x \$100,000 to 10,980 x \$100,000 = **\$450M to \$1,098M annually**

Thus, the **Potential Peak Sales for ruxolitinib in cGVHD** across the US, EU5, China, and Japan are in the range of **\$300M to \$1.1B annually**, depending on penetration and pricing assumptions.

#### Regional Breakdown (Illustrative at 25% Average Share):

- US: ~40% of sales (higher pricing, larger share) = \$120M-\$440M

- EU5: ~35% of sales = \$105M-\$385M
- Japan: ~10% of sales = \$30M-\$110M
- China: ~15% of sales (lower pricing, access barriers) = \$45M-\$165M

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

A 1% share of treated patients corresponds to 1% of the prevalent eligible population (15,000-36,600 patients), i.e., 150-366 patients.

- **Revenue from 1% Share** = 150-366 patients x \$100,000 per patient = **\$15M to \$36.6M annually**

Thus, the **\$ value of a 1% share of treated patients** across these geographies is approximately **\$15M to \$37M per year**.

#### Regional Breakdown of 1% Share Value (Illustrative):

- US: ~\$6M-\$14M (higher pricing)
- EU5: ~\$5M-\$12M
- Japan: ~\$1.5M-\$4M
- China: ~\$2M-\$6M

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## **Key Assumptions and Caveats**

1. **Patient Population:** Estimates are based on general HSCT and cGVHD incidence rates. Real-world data may differ by region due to transplant practices and diagnosis rates.
2. **Pricing:** Costs are approximated and vary significantly by region due to healthcare systems, reimbursement, and discounts.
3. **Penetration:** A 20%-30% share assumes ruxolitinib becomes a standard of care in later-line cGVHD, but competition (e.g., ibrutinib, belumosudil) or access issues could lower this.
4. **Duration of Therapy:** Assumes patients are treated for at least 1 year; actual duration may vary based on response and tolerability.
5. **Peak Sales Timing:** Peak sales are typically reached 5-10 years post-launch, depending on uptake and market dynamics.

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## **Final Answer**

- **Potential Peak Sales for ruxolitinib in cGVHD** (US, EU5, China, Japan):
- At 20% share: **\$300M to \$732M annually**

- At 30% share: **\$450M to \$1.1B annually**
- **\$ Value of 1% Share of Treated Patients:**
- **\$15M to \$37M annually**

These figures are estimates and should be refined with primary market research, actual pricing data, and updated epidemiology figures for cGVHD in these regions. If you have specific data (e.g., exact patient numbers or pricing), I can adjust the calculations accordingly.