

To estimate the **Potential Peak Sales** for eflornithine (IWILFIN) in the indication of high-risk neuroblastoma (HRNB) 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. This involves making assumptions about the target patient population, treatment rates, market share, pricing, and other relevant factors. Since specific data on patient numbers, pricing, and penetration rates for eflornithine in HRNB may not be fully available, I will outline the methodology and use reasonable estimates based on publicly available data and industry standards.

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

High-risk neuroblastoma (HRNB) is a rare pediatric cancer, primarily affecting children under the age of 5. The indication for eflornithine is to reduce the risk of relapse in patients who have demonstrated at least a partial response to prior multiagent, multimodality therapy including anti-GD2 immunotherapy.

### **- Incidence of Neuroblastoma:**

- US: ~700-800 new cases annually, with ~50% classified as high-risk (HRNB), so ~350-400 HRNB cases per year.
- EU5: Incidence is similar to the US, adjusted for population. EU5 population is ~330 million (vs. US ~330 million), so ~350-400 HRNB cases annually.
- China: Population ~1.4 billion, with a higher pediatric population. Incidence of neuroblastoma is ~2,000-2,500 cases annually, with ~50% HRNB, so ~1,000-1,250 cases.
- Japan: Population ~125 million, with ~100-150 neuroblastoma cases annually, ~50-75 HRNB cases.

- **Eligible Patients for Eflornithine:** Not all HRNB patients will be eligible. The indication specifies patients with at least a partial response to prior therapy (including anti-GD2 immunotherapy like dinutuximab). Assuming ~60-70% of HRNB patients achieve at least a partial response and complete prior therapy, we can estimate:

- US: ~210-280 eligible patients annually.
- EU5: ~210-280 eligible patients annually.
- China: ~600-875 eligible patients annually.
- Japan: ~30-50 eligible patients annually.

- **Prevalence vs. Incidence:** Since HRNB treatment and relapse prevention can span multiple years, we also consider prevalent patients (those currently on or eligible for maintenance therapy). Assuming a 3-5 year treatment or follow-up period, the prevalent pool is roughly 3-5 times the annual incidence:

- US: ~630-1,400 prevalent eligible patients.
- EU5: ~630-1,400 prevalent eligible patients.
- China: ~1,800-4,375 prevalent eligible patients.
- Japan: ~90-250 prevalent eligible patients.

For peak sales estimation, we often use the prevalent pool as it reflects the total addressable market at steady state.

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## **Step 2: Estimate Treatment Rate and Market Share**

- **Treatment Rate:** Not all eligible patients will receive eflornithine due to factors like access, physician preference, or reimbursement. Assuming a treatment rate of 50-70% in developed markets (US, EU5, Japan) and 30-50% in China due to cost and access barriers:

- US: 50-70% of ~630-1,400 = ~315-980 treated patients.
- EU5: 50-70% of ~630-1,400 = ~315-980 treated patients.
- China: 30-50% of ~1,800-4,375 = ~540-2,188 treated patients.
- Japan: 50-70% of ~90-250 = ~45-175 treated patients.

- **Market Share for Eflornithine:** The problem specifies a 20-30% share of treated patients for eflornithine. Applying this:

- US: 20-30% of 315-980 = ~63-294 patients.
- EU5: 20-30% of 315-980 = ~63-294 patients.
- China: 20-30% of 540-2,188 = ~108-656 patients.
- Japan: 20-30% of 45-175 = ~9-53 patients.

Total treated patients with eflornithine at peak: **~243-1,297 patients** across all geographies.

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## **Step 3: Estimate Pricing and Annual Cost per Patient**

Eflornithine (IWILFIN) is an orphan drug for a rare disease, so pricing is expected to be high, similar to other orphan drugs in oncology.

- **US Pricing:** Orphan drugs for pediatric cancers often range from \$100,000 to \$300,000 per patient per year. Assume an average of \$200,000/year.

- **EU5 Pricing:** Typically 20-30% lower than the US due to price controls. Assume \$140,000-\$160,000/year (average \$150,000).

- **China Pricing:** Significantly lower due to affordability and reimbursement challenges. Assume \$50,000-\$70,000/year (average \$60,000).

- **Japan Pricing:** Similar to EU5, assume \$140,000-\$160,000/year (average \$150,000).

- **Duration of Therapy:** Eflornithine is likely used as a maintenance therapy to prevent relapse. Assume treatment duration of 1-2 years. For simplicity, use an average annual cost for peak sales calculation, assuming 1 year of treatment at full cost.

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

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

### **- US:**

- Patients: 63-294
- Cost: \$200,000/year
- Peak Sales:  $(63 \times 200,000)$  to  $(294 \times 200,000) = \text{\$12.6M to \$58.8M}$

### **- EU5:**

- Patients: 63-294
- Cost: \$150,000/year
- Peak Sales:  $(63 \times 150,000)$  to  $(294 \times 150,000) = \text{\$9.5M to \$44.1M}$

### **- China:**

- Patients: 108-656
- Cost: \$60,000/year
- Peak Sales:  $(108 \times 60,000)$  to  $(656 \times 60,000) = \text{\$6.5M to \$39.4M}$

### **- Japan:**

- Patients: 9-53
- Cost: \$150,000/year
- Peak Sales:  $(9 \times 150,000)$  to  $(53 \times 150,000) = \text{\$1.4M to \$8.0M}$

### **- Total Peak Sales Across Geographies:**

- Low end:  $\text{\$12.6M (US)} + \text{\$9.5M (EU5)} + \text{\$6.5M (China)} + \text{\$1.4M (Japan)} = \text{\$30.0M}$
- High end:  $\text{\$58.8M (US)} + \text{\$44.1M (EU5)} + \text{\$39.4M (China)} + \text{\$8.0M (Japan)} = \text{\$150.3M}$

**Potential Peak Sales Range for Eflornithine in HRNB: \$30M to \$150M annually** across US, EU5, China, and Japan, assuming 20-30% market share.

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

First, calculate the total number of treated patients (from Step 2, before applying eflornithine's market share) and the revenue per 1% of treated patients.

### **- Total Treated Patients (before market share):**

- US: 315-980

- EU5: 315-980
- China: 540-2,188
- Japan: 45-175
- Total: 1,215-4,323 treated patients

**- 1% of Treated Patients:**

- Low end: 1% of 1,215 = ~12 patients
- High end: 1% of 4,323 = ~43 patients

**- Revenue per 1% Share** (weighted by geography and pricing):

- US: 1% of 315-980 = 3-10 patients × \$200,000 = \$0.6M-\$2.0M
- EU5: 1% of 315-980 = 3-10 patients × \$150,000 = \$0.5M-\$1.5M
- China: 1% of 540-2,188 = 5-22 patients × \$60,000 = \$0.3M-\$1.3M
- Japan: 1% of 45-175 = 0.5-1.8 patients × \$150,000 = \$0.1M-\$0.3M

**- Total \$ Value of 1% Share:**

- Low end: \$0.6M (US) + \$0.5M (EU5) + \$0.3M (China) + \$0.1M (Japan) = **\$1.5M**
- High end: \$2.0M (US) + \$1.5M (EU5) + \$1.3M (China) + \$0.3M (Japan) = **\$5.1M**

**\$ Value of 1% Share of Treated Patients: \$1.5M to \$5.1M annually** across US, EU5, China, and Japan.

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## **Summary**

1. **Potential Peak Sales for Eflornithine (20-30% market share): \$30M to \$150M annually** across the specified geographies.
2. **\$ Value of 1% Share of Treated Patients: \$1.5M to \$5.1M annually** across the specified geographies.

## **Caveats and Assumptions**

- Patient population estimates are based on general incidence/prevalence data for HRNB and may vary based on exact eligibility criteria for eflornithine.
- Pricing assumptions are based on typical orphan drug pricing in oncology; actual pricing for IWILFIN may differ.
- Treatment rates and market share assumptions are speculative and depend on reimbursement, competition, and clinical adoption.
- Peak sales assume steady-state market penetration, which may take several years post-launch to achieve.

If more specific data (e.g., exact patient numbers, pricing, or market dynamics) is available, these estimates can be refined further.