

To estimate the **Potential Peak Sales** for nirogacestat (OGSIVEO) in the indication of desmoid tumors across 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. Since exact data on patient populations, pricing, and market penetration may not be publicly available, I will make reasonable assumptions based on available information, epidemiology data, and typical market dynamics for rare disease drugs. Let's break this down step by step.

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## **\*\*Key Assumptions and Methodology\*\***

1. **Indication:** Nirogacestat is approved for adult patients with progressing desmoid tumors requiring systemic treatment. Desmoid tumors are rare, with an estimated incidence of 2-5 per million people annually.
2. **Patient Population:** We will estimate the number of eligible patients in each geography based on population size and incidence/prevalence data for desmoid tumors.
3. **Market Penetration:** Given the assumption of a 20%-30% share of treated patients, we will calculate peak sales based on this range.
4. **Pricing:** As a novel, first-in-class drug for a rare disease, nirogacestat is likely to be priced at a premium, similar to other orphan drugs. We will assume an annual treatment cost of approximately \$250,000 in the US (a common benchmark for rare disease therapies) and adjust for other regions based on pricing differences (e.g., 50%-70% of US price in EU5 and Japan, and lower in China).
5. **Treatment Duration:** Assume patients remain on therapy for an average of 1-2 years, but for simplicity, we will calculate based on annual costs and assume peak sales reflect steady-state treated patients.
6. **Peak Sales Timing:** Peak sales are typically reached 5-7 years post-launch after market penetration stabilizes.

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## **\*\*Step 1: Estimate Eligible Patient Population\*\***

Desmoid tumors are rare, with an incidence of approximately 2-5 per million people per year. Prevalence (total number of patients at any given time) is harder to estimate but is generally higher than incidence due to the chronic nature of the disease. For simplicity, we will use an incidence of 3 per million and assume a prevalence of 10 per million (accounting for patients living with the disease over several years). Only a subset of these patients will have progressing tumors requiring systemic treatment (assume ~50%).

### **#### Population Estimates (2023)**

- **US:** ~330 million
- **EU5:** ~260 million (France: 65M, Germany: 83M, Italy: 59M, Spain: 47M, UK: 67M)
- **China:** ~1,400 million
- **Japan:** ~125 million

**#### Prevalent Patients (10 per million)**

- **US:**  $330M * 10/1M = 3,300$  patients
- **EU5:**  $260M * 10/1M = 2,600$  patients
- **China:**  $1,400M * 10/1M = 14,000$  patients
- **Japan:**  $125M * 10/1M = 1,250$  patients

**#### Eligible Patients Requiring Systemic Treatment (50% of prevalent patients)**

- **US:**  $3,300 * 0.5 = 1,650$  patients
- **EU5:**  $2,600 * 0.5 = 1,300$  patients
- **China:**  $14,000 * 0.5 = 7,000$  patients
- **Japan:**  $1,250 * 0.5 = 625$  patients
- **Total Eligible Patients:**  $1,650 + 1,300 + 7,000 + 625 = 10,575$  patients

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**\*\*Step 2: Estimate Treated Patients (20%-30% Market Share)\*\***

**- 20% Share:**

- **US:**  $1,650 * 0.2 = 330$  patients
- **EU5:**  $1,300 * 0.2 = 260$  patients
- **China:**  $7,000 * 0.2 = 1,400$  patients
- **Japan:**  $625 * 0.2 = 125$  patients
- **Total:**  $330 + 260 + 1,400 + 125 = 2,115$  patients

**- 30% Share:**

- **US:**  $1,650 * 0.3 = 495$  patients
- **EU5:**  $1,300 * 0.3 = 390$  patients
- **China:**  $7,000 * 0.3 = 2,100$  patients
- **Japan:**  $625 * 0.3 = 188$  patients
- **Total:**  $495 + 390 + 2,100 + 188 = 3,173$  patients

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**\*\*Step 3: Estimate Pricing per Patient per Year\*\***

- **US:** \$250,000 per patient per year (orphan drug benchmark)
- **EU5:** \$175,000 per patient per year (70% of US price due to pricing controls)
- **Japan:** \$175,000 per patient per year (similar to EU5)

- **China:** \$100,000 per patient per year (40% of US price due to lower willingness to pay and pricing negotiations)

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

### **#### At 20% Market Share:**

- **US:** 330 patients \* \$250,000 = \$82.5 million
- **EU5:** 260 patients \* \$175,000 = \$45.5 million
- **China:** 1,400 patients \* \$100,000 = \$140.0 million
- **Japan:** 125 patients \* \$175,000 = \$21.9 million
- **Total Peak Sales (20%):** \$82.5M + \$45.5M + \$140.0M + \$21.9M = **\$290.9 million**

### **#### At 30% Market Share:**

- **US:** 495 patients \* \$250,000 = \$123.8 million
- **EU5:** 390 patients \* \$175,000 = \$68.3 million
- **China:** 2,100 patients \* \$100,000 = \$210.0 million
- **Japan:** 188 patients \* \$175,000 = \$32.9 million
- **Total Peak Sales (30%):** \$123.8M + \$68.3M + \$210.0M + \$32.9M = **\$435.0 million**

**#### Range of Potential Peak Sales: \$290.9 million to \$435.0 million**

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

A 1% share corresponds to 1% of the eligible patients in each geography.

### **#### Eligible Patients for 1% Share:**

- **US:** 1,650 \* 0.01 = 16.5 patients
- **EU5:** 1,300 \* 0.01 = 13.0 patients
- **China:** 7,000 \* 0.01 = 70.0 patients
- **Japan:** 625 \* 0.01 = 6.25 patients

### **#### Revenue for 1% Share:**

- **US:** 16.5 patients \* \$250,000 = \$4.13 million
- **EU5:** 13.0 patients \* \$175,000 = \$2.28 million
- **China:** 70.0 patients \* \$100,000 = \$7.00 million

- **Japan:** 6.25 patients \* \$175,000 = \$1.09 million

- **Total Value of 1% Share:** \$4.13M + \$2.28M + \$7.00M + \$1.09M = **\$14.5 million**

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### **\*\*Final Answers\*\***

1. **Potential Peak Sales for Nirogacestat** in the US, EU5, China, and Japan (assuming 20%-30% market share):

- **Range: \$290.9 million to \$435.0 million annually**

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

- **Total: \$14.5 million annually**

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### **\*\*Caveats and Notes\*\***

- These estimates are based on assumptions about patient numbers, pricing, and market penetration. Actual figures may vary depending on real-world data, reimbursement policies, competition, and treatment duration.

- Desmoid tumors are rare, and patient identification and diagnosis rates may impact the actual treatable population.

- Pricing in China may be lower than assumed due to government negotiations and access programs.

- Peak sales could be higher if nirogacestat gains additional indications or if pricing exceeds assumptions.

If you have access to more specific data (e.g., exact patient numbers, confirmed pricing, or market research), these estimates can be refined further.