

To estimate the **Potential Peak Sales** for **naxitamab-gqgk (DANYELZA)** in the indication of relapsed or refractory high-risk neuroblastoma in the US, EU5 (France, Germany, Italy, Spain, UK), China, and Japan, as well as the **\$ value of a 1% share of treated patients**, we need to follow a structured approach based on available data and reasonable assumptions. Since exact patient numbers, pricing, and market penetration can vary, I will outline the methodology and provide approximate figures based on publicly available information and typical market dynamics for rare diseases like neuroblastoma.

Step 1: Define the Target Patient Population

Neuroblastoma is a rare pediatric cancer, with a specific subset of patients having relapsed or refractory high-risk disease. The indication for naxitamab-gqgk is for pediatric and adult patients with relapsed/refractory high-risk neuroblastoma in the bone or bone marrow who have shown a partial response, minor response, or stable disease to prior therapy.

- **US:** Approximately 700-800 new cases of neuroblastoma are diagnosed annually, with about 50% being high-risk. Of these, a significant portion (around 40-50%) may relapse or become refractory. Assuming ~350 high-risk cases annually, with 40-50% relapsed/refractory, the eligible population could be ~140-175 patients per year. Considering prevalence (patients living with the disease over multiple years), the total addressable population may be higher (~500-700 patients).

- **EU5:** Incidence is similar to the US, adjusted for population. With a combined population of ~330 million (vs. ~330 million in the US), the annual new cases are ~600-700, with ~300 high-risk, and ~120-150 relapsed/refractory annually. Prevalence may be ~400-600 patients.

- **China:** With a population of ~1.4 billion, incidence scales up significantly. Assuming a slightly lower diagnosis rate due to healthcare access, ~2,000-2,500 new cases annually, with ~1,000-1,250 high-risk, and ~400-500 relapsed/refractory. Prevalence may be ~1,500-2,000 patients.

- **Japan:** Population of ~125 million, with ~250-300 new cases annually, ~125-150 high-risk, and ~50-75 relapsed/refractory. Prevalence may be ~150-200 patients.

Total Addressable Population (Prevalence Estimate for Treated Patients):

- US: ~600 patients
- EU5: ~500 patients
- China: ~1,750 patients
- Japan: ~175 patients
- **Total:** ~3,025 patients

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

The problem assumes a 20%-30% share of treated patients for naxitamab-gqgk. Given the rarity of the disease and the unmet need in relapsed/refractory high-risk neuroblastoma, this penetration rate is reasonable, especially as naxitamab is one of the few targeted therapies approved for this indication.

- **At 20% penetration:** ~605 patients treated annually ($3,025 * 0.2$)
- **At 30% penetration:** ~908 patients treated annually ($3,025 * 0.3$)

Breakdown by Geography (at 20%-30% penetration):

- US: 120-180 patients
- EU5: 100-150 patients
- China: 350-525 patients
- Japan: 35-53 patients

Step 3: Pricing of Naxitamab-gqqgk

Naxitamab is a high-cost therapy due to its orphan drug status and targeted indication. Based on available information:

- In the US, the cost of naxitamab is approximately **\$20,000 per vial**, with a typical treatment course requiring multiple vials over several cycles. A full course of treatment may cost ~\$500,000-\$1,000,000 per patient annually, depending on dosing and duration.
- In EU5 and Japan, pricing may be slightly lower due to healthcare system negotiations (~70%-80% of US price, or ~\$350,000-\$800,000 per patient).
- In China, pricing is often significantly lower due to market access and affordability constraints (~30%-50% of US price, or ~\$150,000-\$500,000 per patient).

Assumed Average Annual Cost per Patient:

- US: \$750,000
- EU5: \$600,000
- Japan: \$600,000
- China: \$300,000

Step 4: Calculate Potential Peak Sales

Peak sales are calculated as the number of treated patients (at 20%-30% penetration) multiplied by the average annual cost per patient in each geography.

At 20% Penetration:

- **US:** 120 patients * \$750,000 = **\$90 million**
- **EU5:** 100 patients * \$600,000 = **\$60 million**
- **China:** 350 patients * \$300,000 = **\$105 million**
- **Japan:** 35 patients * \$600,000 = **\$21 million**

- **Total Peak Sales (20%):** $\$90\text{M} + \$60\text{M} + \$105\text{M} + \$21\text{M} = \$276 \text{ million}$

At 30% Penetration:

- **US:** 180 patients * $\$750,000 = \135 million

- **EU5:** 150 patients * $\$600,000 = \90 million

- **China:** 525 patients * $\$300,000 = \157.5 million

- **Japan:** 53 patients * $\$600,000 = \31.8 million

- **Total Peak Sales (30%):** $\$135\text{M} + \$90\text{M} + \$157.5\text{M} + \$31.8\text{M} = \$414.3 \text{ million}$

Potential Peak Sales Range: \$276 million to \$414 million annually

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

A 1% share of treated patients corresponds to 1% of the total addressable population (~3,025 patients), which is ~30 patients.

Value of 1% Share (30 patients):

- **US:** $(600 * 0.01) = 6 \text{ patients} * \$750,000 = \$4.5 \text{ million}$

- **EU5:** $(500 * 0.01) = 5 \text{ patients} * \$600,000 = \$3 \text{ million}$

- **China:** $(1,750 * 0.01) = 17.5 \text{ patients} * \$300,000 = \$5.25 \text{ million}$

- **Japan:** $(175 * 0.01) = 1.75 \text{ patients} * \$600,000 = \$1.05 \text{ million}$

- **Total Value of 1% Share:** $\$4.5\text{M} + \$3\text{M} + \$5.25\text{M} + \$1.05\text{M} = \$13.8 \text{ million}$

Final Answer:

1. Potential Peak Sales for Naxitamab-gqqk (20%-30% penetration):

- Range: **\$276 million to \$414 million annually** across the US, EU5, China, and Japan.

- Breakdown:

- US: $\$90\text{M} - \135M

- EU5: $\$60\text{M} - \90M

- China: $\$105\text{M} - \157.5M

- Japan: $\$21\text{M} - \31.8M

2. \$ Value of 1% Share of Treated Patients:

- Total: **\$13.8 million annually**

- Breakdown:
- US: \$4.5M
- EU5: \$3M
- China: \$5.25M
- Japan: \$1.05M

Note: These estimates are based on assumptions about patient population, pricing, and market penetration. Actual figures may vary based on real-world data, reimbursement policies, competition, and market access challenges, especially in China. For more precise calculations, detailed epidemiology data, pricing agreements, and market research would be required.