To estimate the **Potential Peak Sales** for azacitidine (Vidaza) in the indication of juvenile myelomonocytic leukemia (JMML) 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 exact patient numbers, pricing, and market penetration rates may not be fully available, I will outline the methodology and make reasonable assumptions based on publicly available information and typical market analysis frameworks for rare diseases like JMML.

## Step 1: Key Assumptions and Methodology

1. **Indication and Patient Population**: JMML is a rare pediatric leukemia with an estimated incidence of 1.2 cases per million children per year. It primarily affects children under the age of 4. We will estimate the prevalent patient population in each geography based on population size and incidence/prevalence rates.

2. **Share of Treated Patients**: As per the query, we assume azacitidine captures 20% to 30% of treated patients in this indication.

3. **Pricing**: Azacitidine is a high-cost drug for rare diseases. Pricing varies by region, but we will assume an annual treatment cost based on typical pricing for oncology drugs in each geography (e.g., $100,000–$150,000 per patient per year in the US, lower in other regions due to price controls).

4. **Peak Sales**: Peak sales are typically achieved 5–10 years after launch, assuming maximum market penetration.

5. **Geographies**: US, EU5 (combined), China, and Japan.

## Step 2: Estimating Patient Population for JMML

JMML is extremely rare, and prevalence is often used for rare diseases to estimate the total treatable population. Incidence is approximately 1.2 per million children annually, and prevalence can be estimated as a multiple of incidence (e.g., 3–5 years of survival, adjusted for mortality).

- **US**: Population ~330 million; pediatric population (0–14 years) ~60 million. Incidence = 1.2 per million = ~72 new cases/year. Prevalence (assuming 3-year survival) = ~216 patients.

- **EU5**: Combined population ~260 million; pediatric population ~45 million. Incidence = ~54 new cases/year. Prevalence = ~162 patients.

- **China**: Population ~1.4 billion; pediatric population ~240 million. Incidence = ~288 new cases/year. Prevalence = ~864 patients.

- **Japan**: Population ~125 million; pediatric population ~15 million. Incidence = ~18 new cases/year. Prevalence = ~54 patients.

Total prevalent patients across geographies = 216 (US) + 162 (EU5) + 864 (China) + 54 (Japan) = **1,296 patients**.

## Step 3: Estimating Treated Patients

Not all prevalent patients may be treated due to diagnosis rates, access to healthcare, and treatment eligibility. For rare diseases, we assume ~80% of prevalent patients are diagnosed and eligible for treatment.

- Total treatable patients = 1,296 * 80% = **1,037 patients**.

- Distribution:

- US: 216 * 80% = 173 patients

- EU5: 162 * 80% = 130 patients

- China: 864 * 80% = 691 patients

- Japan: 54 * 80% = 43 patients

## Step 4: Market Share and Treated Patients for Azacitidine

Assuming azacitidine captures 20% to 30% of treated patients:

- At 20% share: 1,037 * 20% = **207 patients**

- At 30% share: 1,037 * 30% = **311 patients**

Distribution by geography (proportional):

- **US**: 173 patients * 20%–30% = 35–52 patients

- **EU5**: 130 patients * 20%–30% = 26–39 patients

- **China**: 691 patients * 20%–30% = 138–207 patients

- **Japan**: 43 patients * 20%–30% = 9–13 patients

## Step 5: Pricing per Patient per Year

Annual treatment cost for azacitidine varies by region due to healthcare systems and pricing negotiations. Assumptions:

- **US**: $120,000 per patient per year (high due to lack of price controls)

- **EU5**: $80,000 per patient per year (lower due to negotiated pricing)

- **China**: $40,000 per patient per year (significantly lower due to pricing policies and generics)

- **Japan**: $90,000 per patient per year (high but slightly lower than US)

## Step 6: Potential Peak Sales Calculation

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

#### At 20% Market Share:

- **US**: 35 patients * $120,000 = **$4.2 million**

- **EU5**: 26 patients * $80,000 = **$2.1 million**

- **China**: 138 patients * $40,000 = **$5.5 million**

- **Japan**: 9 patients * $90,000 = **$0.8 million**

- **Total Peak Sales (20% share)**: $4.2M + $2.1M + $5.5M + $0.8M = **$12.6 million**

#### At 30% Market Share:

- **US**: 52 patients * $120,000 = **$6.2 million**

- **EU5**: 39 patients * $80,000 = **$3.1 million**

- **China**: 207 patients * $40,000 = **$8.3 million**

- **Japan**: 13 patients * $90,000 = **$1.2 million**

- **Total Peak Sales (30% share)**: $6.2M + $3.1M + $8.3M + $1.2M = **$18.8 million**

Thus, **Potential Peak Sales** for azacitidine in JMML across these geographies range from **$12.6 million (20% share)** to **$18.8 million (30% share)** annually.

## Step 7: $ Value of 1% Share of Treated Patients

A 1% share of treated patients corresponds to 1% of the 1,037 treatable patients = **10.37 patients**.

Value of 1% share by geography:

- **US**: (173 * 1%) * $120,000 = 1.73 patients * $120,000 = **$0.21 million ($210,000)**

- **EU5**: (130 * 1%) * $80,000 = 1.3 patients * $80,000 = **$0.10 million ($104,000)**

- **China**: (691 * 1%) * $40,000 = 6.91 patients * $40,000 = **$0.28 million ($276,400)**

- **Japan**: (43 * 1%) * $90,000 = 0.43 patients * $90,000 = **$0.04 million ($38,700)**

- **Total Value of 1% Share**: $0.21M + $0.10M + $0.28M + $0.04M = **$0.63 million ($630,100)**

## Final Answer

1. **Potential Peak Sales for Azacitidine in JMML** (assuming 20%–30% share of treated patients):

- **US, EU5, China, Japan combined**: **$12.6 million (20% share) to $18.8 million (30% share)** annually.

- Breakdown:

- US: $4.2M–$6.2M

- EU5: $2.1M–$3.1M

- China: $5.5M–$8.3M

- Japan: $0.8M–$1.2M

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

- **US, EU5, China, Japan combined**: **$0.63 million ($630,100)**

- Breakdown:

- US: $210,000

- EU5: $104,000

- China: $276,400

- Japan: $38,700

## Notes

- These estimates are based on assumptions about prevalence, treatment rates, market share, and pricing. Real-world data may differ due to factors like competition, reimbursement policies, and patient access.

- JMML is a very rare disease, so peak sales are relatively low compared to drugs for more common indications.

- If azacitidine is used in combination therapies or other indications (e.g., MDS or AML), sales could be significantly higher, but this analysis focuses solely on JMML.