To estimate the **Potential Peak Sales** for avapritinib (Ayvakit) in the indication of advanced systemic mastocytosis (AdvSM) 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 exact data on patient populations, pricing, and market penetration may not be publicly available, I will outline the methodology and use reasonable assumptions based on available information and industry standards. The final numbers are illustrative and should be validated with real-world data.

Step 1: Define the Target Patient Population for AdvSM

Advanced systemic mastocytosis (AdvSM) is a rare disease with subtypes including aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL). The prevalence of systemic mastocytosis (SM) is estimated to be around 1 in 10,000 to 20,000 people, with AdvSM representing a smaller subset (approximately 5-10% of SM cases). For simplicity, we will estimate the prevalence and patient population in each geography.

- **US**: Population ~330 million. Prevalence of SM ~16,500-33,000 patients. AdvSM (5-10%) ~825-3,300 patients.

- **EU5**: Combined population ~260 million. Prevalence of SM ~13,000-26,000 patients. AdvSM ~650-2,600 patients.

- **China**: Population ~1.4 billion. Prevalence of SM ~70,000-140,000 patients. AdvSM ~3,500-14,000 patients. (Note: Diagnosis rates may be lower due to healthcare access.)

- **Japan**: Population ~125 million. Prevalence of SM ~6,250-12,500 patients. AdvSM ~312-1,250 patients.

Assumption: Let's take the midpoint of these ranges for AdvSM patients:

- US: ~2,000 patients

- EU5: ~1,600 patients

- China: ~8,750 patients (adjusted down for lower diagnosis rates, assume 50% diagnosed = ~4,375)

- Japan: ~780 patients

Total AdvSM Patient Population: ~2,000 (US) + 1,600 (EU5) + 4,375 (China) + 780 (Japan) = **8,755 patients**.

Step 2: Estimate the Percentage of Treated Patients

Not all diagnosed patients may receive treatment due to factors like disease severity, access to healthcare, or physician/patient preference. Assuming 70-80% of AdvSM patients are treated:

- **Treated Patients**: ~75% of 8,755 = **6,566 patients**.

Given the query assumes a **20-30% share of treated patients** for avapritinib, we calculate:

- **Avapritinib-treated patients (midpoint 25%)**: 25% of 6,566 = **1,642 patients**.

Breakdown by Geography (assuming proportional distribution):

- US: 2,000 / 8,755 * 1,642 = ~375 patients

- EU5: 1,600 / 8,755 * 1,642 = ~300 patients

- China: 4,375 / 8,755 * 1,642 = ~820 patients

- Japan: 780 / 8,755 * 1,642 = ~147 patients

Step 3: Estimate Annual Cost of Treatment

Avapritinib (Ayvakit) is a targeted therapy for a rare disease, so pricing is high. Based on pricing for similar rare disease drugs and public information:

- **US**: Annual cost per patient ~$300,000–$400,000 (assume $350,000).

- **EU5**: Annual cost ~$200,000–$300,000 (assume $250,000 due to price controls).

- **China**: Annual cost ~$100,000–$150,000 (assume $125,000 due to lower pricing and access issues).

- **Japan**: Annual cost ~$250,000–$350,000 (assume $300,000, similar to US but slightly discounted).

Step 4: Calculate Potential Peak Sales

Peak sales are calculated as the number of treated patients in each geography multiplied by the annual cost per patient.

- **US**: 375 patients * $350,000 = **$131.25 million**

- **EU5**: 300 patients * $250,000 = **$75 million**

- **China**: 820 patients * $125,000 = **$102.5 million**

- **Japan**: 147 patients * $300,000 = **$44.1 million**

Total Potential Peak Sales (25% share): $131.25M + $75M + $102.5M + $44.1M = **$352.85 million annually**.

- **Range for 20-30% share**:

- 20% share (1,313 patients): ~$282.28 million

- 30% share (1,970 patients): ~$423.42 million

Thus, **Potential Peak Sales Range**: **$282M to $423M annually**, with a midpoint of **~$353M**.

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

A 1% share of treated patients corresponds to 1% of the 6,566 treated patients = **65.66 patients**.

Breakdown by Geography (proportional):

- US: 2,000 / 8,755 * 65.66 = ~15 patients
- EU5: 1,600 / 8,755 * 65.66 = ~12 patients
- China: 4,375 / 8,755 * 65.66 = ~33 patients
- Japan: 780 / 8,755 * 65.66 = ~6 patients

$ Value of 1% Share:

- US: 15 patients * $350,000 = **$5.25 million**
- EU5: 12 patients * $250,000 = **$3 million**
- China: 33 patients * $125,000 = **$4.125 million**
- Japan: 6 patients * $300,000 = **$1.8 million**

Total $ Value of 1% Share: $5.25M + $3M + $4.125M + $1.8M = **$14.175 million annually**.

Final Answer

1. **Potential Peak Sales for Avapritinib in AdvSM (20-30% share of treated patients):**

- Range: **$282 million to $423 million annually**
- Midpoint (25% share): **~$353 million annually**

Breakdown by Geography (25% share):

- US: ~$131.25 million
- EU5: ~$75 million
- China: ~$102.5 million
- Japan: ~$44.1 million

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

- Total: **$14.175 million annually**

Breakdown by Geography:

- US: ~$5.25 million
- EU5: ~$3 million

- China: ~$4.125 million

- Japan: ~$1.8 million

Note: These estimates are based on assumptions about patient population, treatment rates, market share, and pricing. Real-world data (e.g., actual diagnosed patients, reimbursement policies, and competitor dynamics) would refine these numbers. Additionally, peak sales may take several years to achieve due to market penetration timelines, and pricing may vary based on negotiations with payers. For precise figures, consult primary market research or financial reports from Blueprint Medicines.