To calculate the **Potential Peak Sales** for remestemcel-L-rknd (Ryoncil) in the indication of steroid-refractory acute graft versus host disease (SR-aGVHD) in pediatric patients 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 specific data such as exact patient numbers, pricing, and penetration rates are not provided, I will make reasonable assumptions based on publicly available information, epidemiology data, and market analysis for rare diseases and cell therapies. Let's break this down step by step.

Step 1: Define the Target Patient Population

SR-aGVHD is a rare and severe complication of allogeneic hematopoietic stem cell transplantation (HSCT), primarily affecting pediatric patients in this indication. We will estimate the number of eligible patients in each geography.

- **US**: Approximately 8,000-10,000 HSCTs are performed annually in the US. About 50-60% of patients develop aGVHD, and roughly 30-40% of those become steroid-refractory. For pediatric patients (a subset), this translates to ~300-500 eligible patients per year.

- **EU5**: The EU5 performs around 15,000-20,000 HSCTs annually. Using similar proportions, the pediatric SR-aGVHD population is estimated at ~600-800 patients per year.

- **China**: China performs an estimated 10,000-15,000 HSCTs annually. Pediatric SR-aGVHD cases are estimated at ~400-600 patients per year.

- **Japan**: Japan performs ~5,000 HSCTs annually. Pediatric SR-aGVHD cases are estimated at ~150-250 patients per year.

Total estimated pediatric SR-aGVHD patients per year:

- US: ~400 (midpoint)

- EU5: ~700 (midpoint)

- China: ~500 (midpoint)

- Japan: ~200 (midpoint)

- **Global Total (these geographies)**: ~1,800 patients per year

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

Given the assumption of a 20%-30% share of treated patients, we will calculate the number of patients treated with remestemcel-L-rknd at peak sales.

- **US**: 400 patients × 20%-30% = 80-120 patients

- **EU5**: 700 patients × 20%-30% = 140-210 patients

- **China**: 500 patients × 20%-30% = 100-150 patients

- **Japan**: 200 patients × 20%-30% = 40-60 patients

- **Total Treated Patients at Peak**: 360-540 patients

Step 3: Pricing Assumptions

Cell therapies like remestemcel-L-rknd are expensive due to manufacturing complexity and the rare disease indication. Pricing will vary by geography due to differences in healthcare systems and reimbursement.

- **US**: Estimated price per treatment ~$200,000-$300,000 (based on comparable therapies like Kymriah or Yescarta, adjusted for indication and pediatric focus).

- **EU5**: Estimated price ~$150,000-$250,000 (lower due to pricing negotiations and public health systems).

- **Japan**: Estimated price ~$150,000-$250,000 (similar to EU5, with high reimbursement potential for rare diseases).

- **China**: Estimated price ~$100,000-$150,000 (lower due to affordability constraints and emerging market dynamics).

For simplicity, we'll use midpoint pricing:

- US: $250,000 per patient

- EU5: $200,000 per patient

- Japan: $200,000 per patient

- China: $125,000 per patient

Step 4: Calculate Potential Peak Sales

Peak sales are calculated by multiplying the number of treated patients by the price per treatment in each geography.

At 20% Market Share:

- **US**: 80 patients × $250,000 = $20 million

- **EU5**: 140 patients × $200,000 = $28 million

- **China**: 100 patients × $125,000 = $12.5 million

- **Japan**: 40 patients × $200,000 = $8 million

- **Total Peak Sales (20% share)**: $68.5 million

At 30% Market Share:

- **US**: 120 patients × $250,000 = $30 million

- **EU5**: 210 patients × $200,000 = $42 million

- **China**: 150 patients × $125,000 = $18.75 million

- **Japan**: 60 patients × $200,000 = $12 million

- **Total Peak Sales (30% share)**: $102.75 million

Potential Peak Sales Range: **$68.5 million to $102.75 million annually** across the US, EU5, China, and Japan.

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

A 1% share of treated patients corresponds to 1% of the total eligible patient population in each geography. We calculate the number of patients and multiply by the price per treatment.

- **US**: 400 patients × 1% = 4 patients × $250,000 = $1 million

- **EU5**: 700 patients × 1% = 7 patients × $200,000 = $1.4 million

- **China**: 500 patients × 1% = 5 patients × $125,000 = $0.625 million

- **Japan**: 200 patients × 1% = 2 patients × $200,000 = $0.4 million

- **Total $ Value of 1% Share**: $3.425 million

$ Value of 1% Share of Treated Patients: **$3.425 million annually** across the US, EU5, China, and Japan.

Final Answer

1. **Potential Peak Sales for remestemcel-L-rknd** in SR-aGVHD (pediatric) with a 20%-30% share of treated patients:

- **$68.5 million to $102.75 million annually** across the US, EU5, China, and Japan.

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

- **$3.425 million annually** across the US, EU5, China, and Japan.

Caveats and Assumptions

- Patient population estimates are based on general HSCT and aGVHD epidemiology data; actual numbers may vary.

- Pricing assumptions are based on comparable cell therapies and may differ based on reimbursement, competition, and market access.

- Peak sales assume steady-state penetration; actual sales may take years to reach peak due to regulatory approvals, market adoption, and manufacturing constraints.

- The analysis assumes no significant competition or alternative therapies capturing market share during the peak sales period.

If more specific data (e.g., exact patient numbers, pricing, or penetration rates) are available, these estimates can be refined.