

Months-Not-Years: Fast-Tracking Pediatric Orphan Drugs into Greater China and SE Asia

Childhood doesn't wait for approvals



ACA Pharma



WUZHOU
DROGA

Outcomes deteriorate with every month lost

Pediatric rare-disease patients face long diagnostic and access delays



Real case (DMD,
Xi'an, 2025)

Direct Hospital Procurement → Deflazacort Initiated

Patient: Male, 4y; DMD (DMD c.358-1G>A); positive Gower's; CK >20,000 U/L.

Center: Northwest Women's & Children's Hospital, Xi'an (Shaanxi).

Work-up: 2025 genetic confirmation; MRI consistent with muscular dystrophy; elevated cardiac enzymes.

Action: Hospital enrolled via Group NPP / direct procurement; deflazacort 6 mg/day initiated; PV & follow-up established.

De-identified case for illustration; no efficacy claims. Deflazacort imported under national permit to a designated hospital.

Approval ≠ Access

Why proven therapies miss their China/SE Asia window



Composite case for illustration; not an actual patient.

- Registration-first trap. Full local registration can mean new trials, new spend, new delays—even for proven therapies.
- Fragmented hospital demand, import permits, PV, QA, and cold-chain add risk and distract lean teams.
- Delay invites fast followers and local competitors, eroding first-mover KOLs, data moats, and pricing power.
- Capital & geopolitics. Most biotechs can't—or won't—plant fixed assets in-country given risk, compliance burden, and irreversible cost.

How Big Is the Need?

65–100M people across China & SE Asia live with a rare disease



- China: ~20–54M people live with a rare disease (range across major estimates).
- Childhood onset: ~50% of rare diseases begin in childhood; ~80% are genetic.
- SE Asia: ~45M people affected (~9% of the region).
- China registry signal: 63,000+ patients across 160+ diseases recorded in the national NRDRS (and growing)—a window into the growing scale of the problem.

Dual Pathways for Pediatric & Orphan Drugs

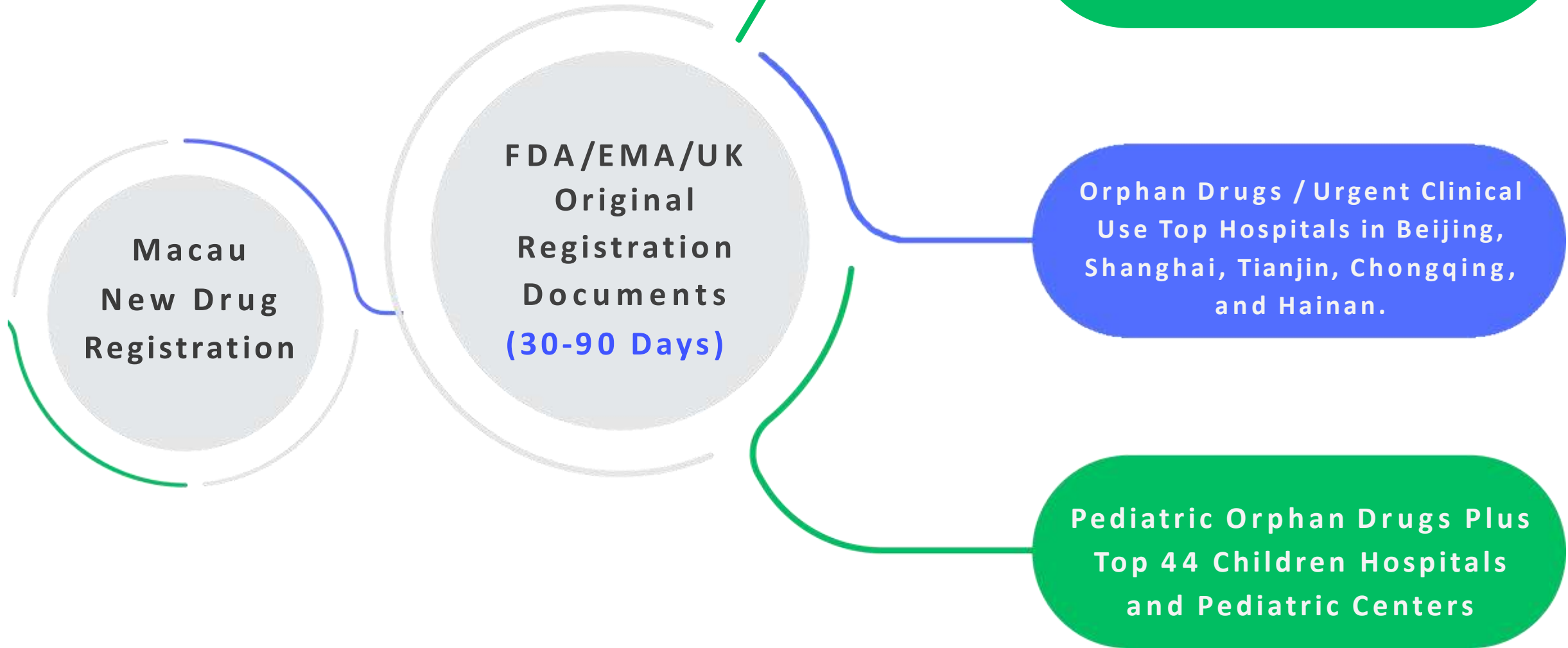
Macau Fast-Track (30–90 Days)

- Rapid approval in 1–3 months
- No NMPA registration or clinical trials
- Direct access to 45 hospitals in GBA (120M pop)
- RWE speeds China national approval:
 - Pediatric orphan: 3–6 months
 - Clinically urgent: 6–12 months
- 80+ drugs and devices registered

Direct Hospital Group Procurement

- 44 top pediatric hospitals across China
- Covers 95% of pediatric rare disease patients
- Hospitals apply for long-term import permits
- No exclusivity, no registration required
- Proven case: Deflazacort for DMD now used in 95% of pediatric hospitals
- Simple purchase + confidentiality agreement to start

Macau New Drug Registration Process

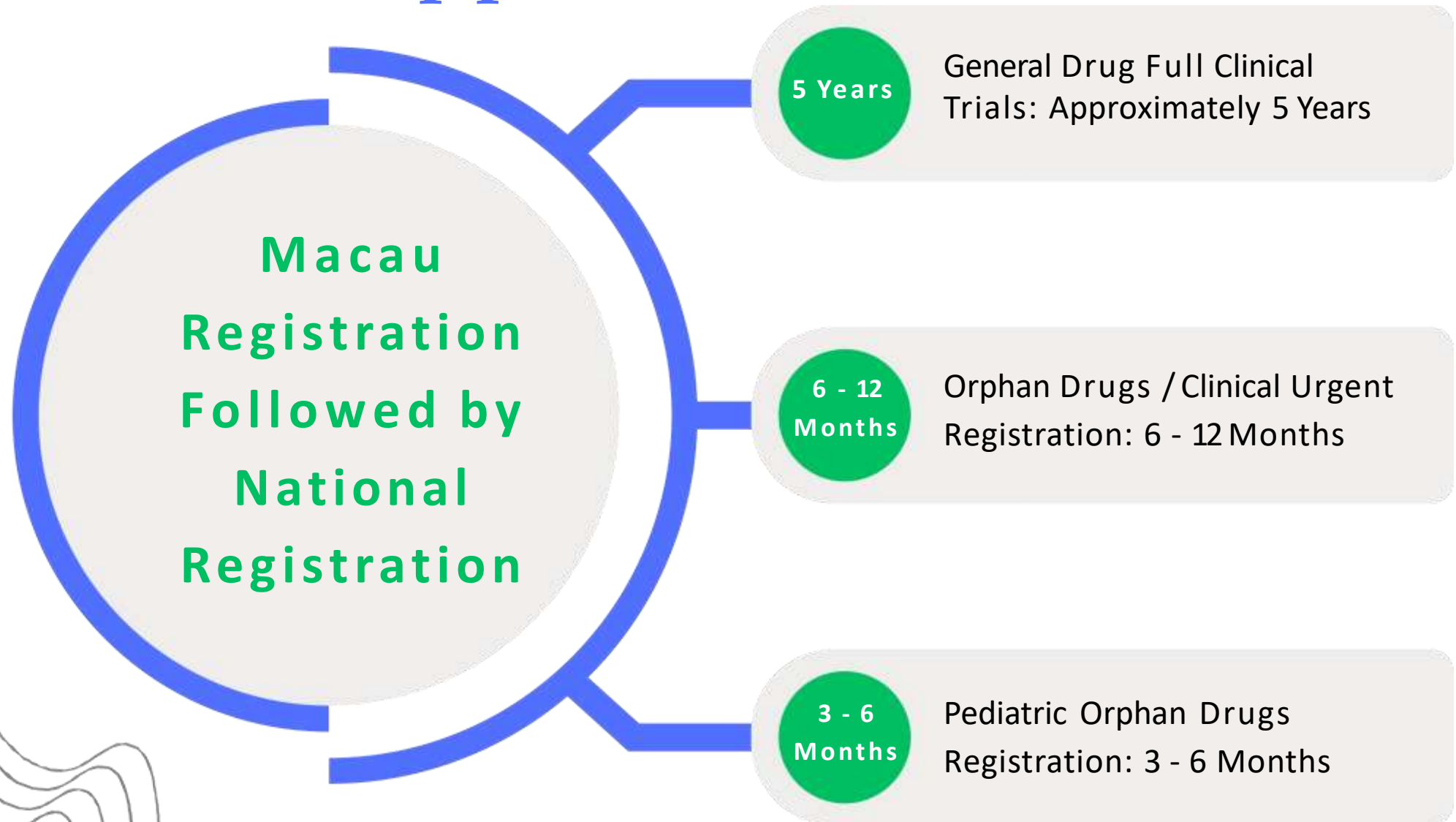


Macau Checklist

<10 items

- **Minimal Documentation:** Only essential documents are needed, such as the Letter of Authorization and Certificate of Pharmaceutical Product (CPP), along with electronic copies of key certificates like GMP.
- **Detailed Submission:** Includes CTD Modules (2-5), a Risk Management Plan, and a Certificate of Analysis (COA).
- **Simple Drug Sample Requirements:** Only one sample box is required, which should contain the medication and an instruction booklet.

National Registration Process After Macau Approval





Multiple Paths to Affordability

Local Insurance in Macau, Singapore & HK

Products are covered by local government insurance post-registration, enhancing affordability and uptake.

Self-Pay in the Greater Bay Area and China

Sales proceed on a self-pay basis in economically vibrant regions, facilitating immediate market entry and revenue before national registration.

National Insurance Inclusion

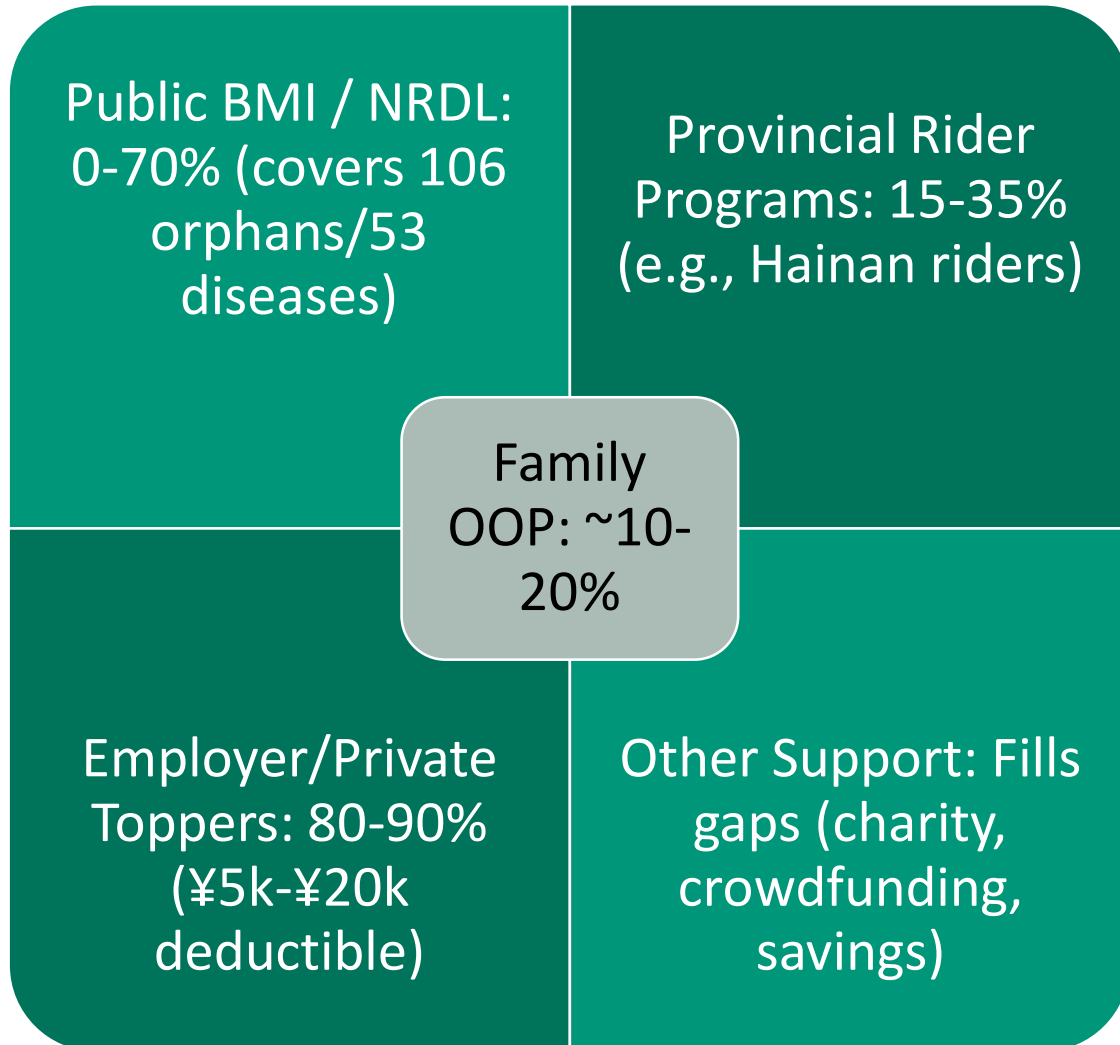
Entry into national medical insurance post-national registration increases accessibility, significantly boosting sales volumes despite lower unit prices.

Private Insurance Toppers in Mainland China

Growing private supplemental plans in Tier 1/2 cities (20-25% urban workforce) cover 80-90% of off-NRDL orphan drugs, bridging affordability gaps.

Bridging the Cost Gap for Rare Diseases

China's Multi-Layered Insurance Approach



- 20-25% of urban workers access private toppers for 80-90% orphan drug coverage.
- Premiums at \$500-2k/year beat US rates, driven by state-led pricing.
- SOE/government plans boost public coverage to 80-90% for listed drugs.
- 30-40% savings rate and crowdfunding, which can raise 50k-500k RMB per case, help fill the gap.

Direct Hospital Procurement

No exclusivity. No registration. Just supply.

- Proven Deflazacort 2-year experience (Macau 30–90 day pathway).
- 44 top pediatric hospitals & leading medical centers, covering >95% of rare disease patients in China.
- Hospitals apply directly for long-term national import permits, bypassing registration and clinical trials.
- Skip registration entirely; we execute end-to-end through our U.S. wholesaler license and local China/Singapore entities.

Requirements:

- A direct supply agreement with originator.
- A confidentiality agreement to protect hospital and patient data.



Your Macau Fast-Track Partner

Who we are & how we help

New York headquartered CSO with licensed entities in Macau, Hong Kong and Singapore handling every step from registration, marketing, distribution to sales, under the guidance of former neurologist Mike Zhou and bilingual teams with 30+ years of China pharma experience.

Execution Network Across China & SE Asia

With over 30 years of experience in the Chinese pharmaceutical industry, we have established a robust network, including highly skilled local teams and extensive sales networks for specialized medicines throughout China and SE Asia. Our advisory team includes renowned experts in various specialties and rare diseases, allowing us to rapidly introduce any Macau-registered drug to leading hospitals. Our team possesses expertise in sales, distribution, and clinical operations, ensuring effective market entry and commercialization.



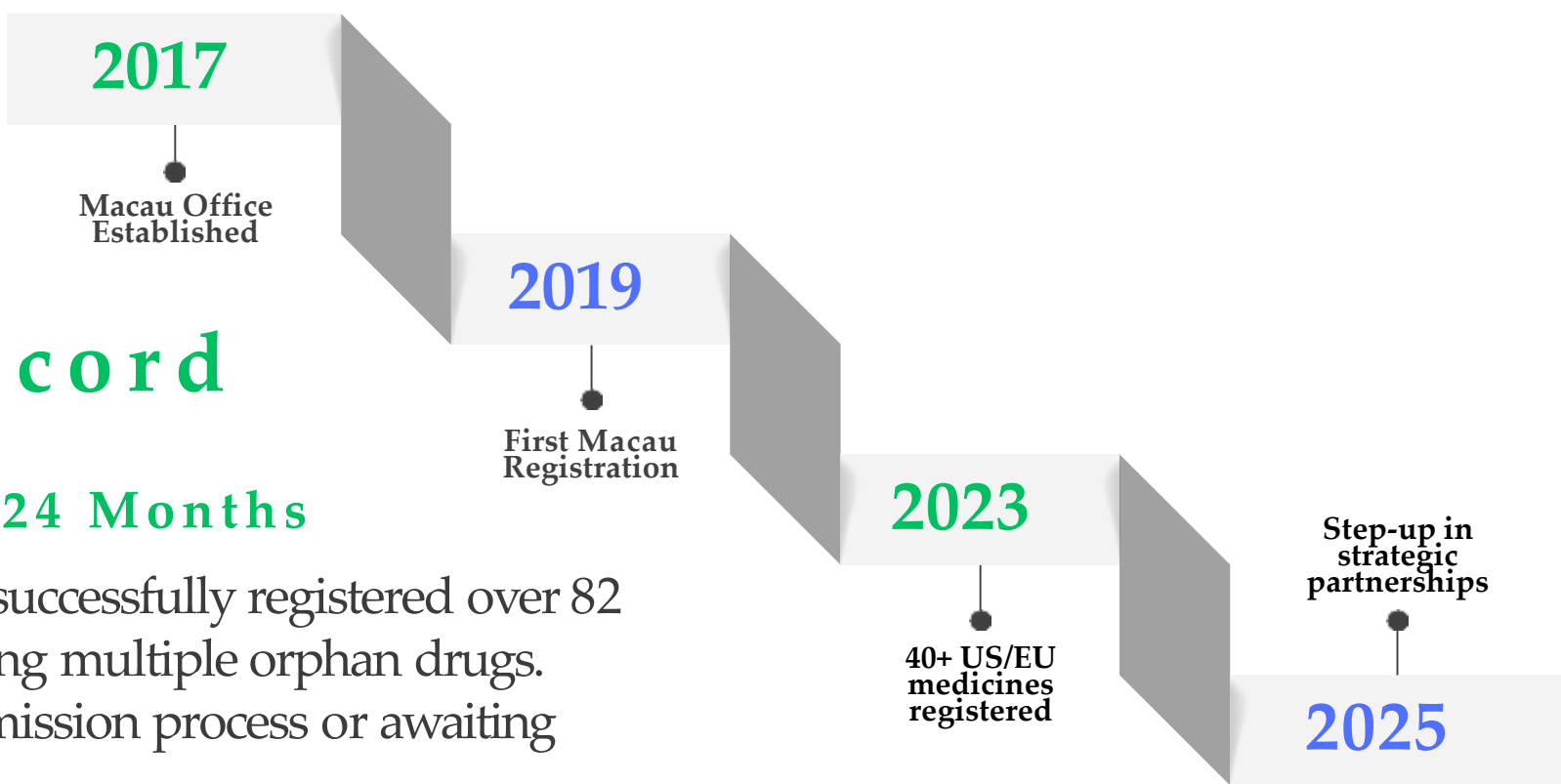
Proven Track Record

Execution Highlights: Last 24 Months

In the past two years, our Macau team successfully registered over 82 US and European medications, including multiple orphan drugs. Currently, 7 more drugs are in the submission process or awaiting approval.

Strategic Partnerships That Move Product

- Our office in Macau is also a proud partner and supplier to China Resources Pharmaceutical Group, one of the Fortune Global 500 companies.
- This collaboration has significantly enhanced our market access, distribution, and logistics capabilities in China's state-owned enterprise-driven market.



Simple CSO Partnership Model

Simple CSO Partnership for Originators

- We fund & execute upfront work: registration, import/logistics, distribution, promotion.
- You retain IP & control; transparent commercial terms with post-launch cost recovery.
- No upfront capital required; rapid time-to-market via Macau Fast-Track (HK/SG in parallel).

What you get

- Speed & certainty: repeatable playbook with regulator-trusted team.
- Scale on day one: licensed warehouses (Macau/HK) & 500+ reps across Mainland; SOE distributor partnerships.
- Single accountable partner: US-HQ contracting, local execution.

