L.E.K.

Special Report

Weighing the Trade-offs of a Direct Presence in Japan's Rare and Orphan Drug Market

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Contents

Summary

Japan has long sought to foster an attractive market for orphan drugs, with specific provisions made to encourage the development of therapies for rare and orphan diseases as early as 1972.[1] The measures and incentives that followed made the orphan and rare model economically viable in Japan, yet companies operating in the space typically addressed the market through local partners rather than establishing local operations themselves. In recent years, however, there has been a marked uptick in the number of rare and orphan companies establishing direct presences in Japan to steward their pipelines through development and to the market, with many more considering doing the same.

Why is this happening? And what does it mean for executives and rare and orphan-focused biopharma companies considering their international strategy?

- **Japan in general has strong fundamentals that make it an attractive market for rare and orphan companies to set up a direct presence.** These include sizable underlying patient populations, relatively attractive pricing, favorable market access, a streamlined regulatory system, and generally strong awareness of rare and orphan diseases, as well as supporting infrastructure to aid diagnosis, treatment and ongoing management of patients.
- **As such, for many rare and orphan companies, a direct presence in Japan is a viable option, even at the point of first launch**, and a means to capture the significant value offered by the Japan market. It is all the more so when you consider the benefits of avoiding entanglement with a partner and the challenges of managing relationships with potential licensing partners.
- **But setting up a direct presence in Japan is not for everyone.** Some disease areas and molecule types are going to face unfavorable volume and/or pricing dynamics that will make the business case for direct entry untenable; companies may lack the pipeline depth to warrant a direct presence; some companies will simply struggle to find the bandwidth to make Japan a priority relative to other geographies.
- **The market landscape is also in flux, which inevitably entails risk in decision-making.** Pricing and access are often the greatest concerns, although the downside for the rare and orphan market appears much less than in other major markets. For novel molecules, especially for gene and cell therapies, the regulatory landscape is still taking shape and, as with other major markets, the reimbursement system does not yet seem well suited to pay for transformative, one-time cures.
- **As such, careful analysis and planning are necessary to guide a thoughtful entry strategy and subsequent operationalization.**

For further information on this Special Report and its findings, please contact lifesciences@lek.com.

Background on increasing interest in Japan among rare and orphan biopharma companies

For all but the largest pharmaceutical companies, the typical Japan strategy had until recently been to license development and commercial rights to a Japanese counterpart and focus attention elsewhere. Some midsize companies — such as UCB and Lundbeck — have experimented with "Japan-lite" entry models whereby they have gradually increased their commitment over time, but in general, companies of an equivalent or smaller size have tended to keep away.

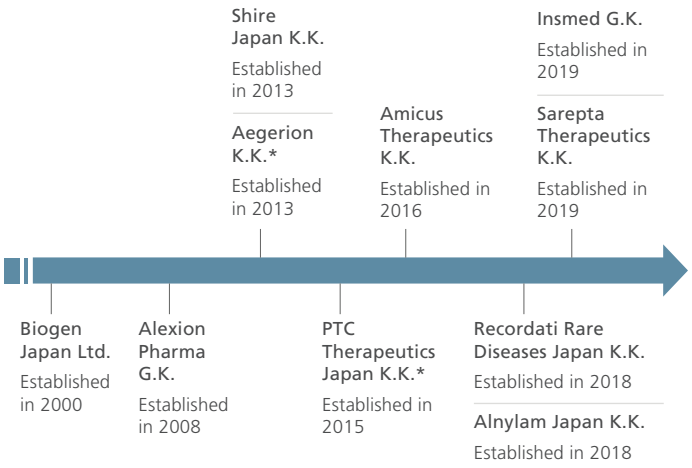
Around 10 years or so ago, this trend began to shift, most notably for companies focused on rare and orphan diseases. Larger companies, such as Biogen and Shire, which both had a long presence in other international markets, established Japan subsidiaries in the early 2010s in support of their rare and orphan assets. Subsequently, smaller companies, starting with Alexion and now including others such as Alnylam, Amicus, Insmed and Recordati, have set up an on-the-ground presence with the intent of developing and commercializing their portfolios in Japan by themselves. The rate of entry has been increasing as the strategy has become increasingly validated, with many rare and orphan companies prioritizing Japan second only to the U.S. (see Figure 1).

What is behind this trend? In part, it is the consequence of regulatory harmonization with other major geographies and relatively attractive pricing. However, other compelling factors in play explain Japan's pull on rare and orphan drugmakers. All the same, the market is not without its challenges, and some of these are expected to become increasingly prominent in the coming years, especially in the arena of pricing and access (see Figure 2).

As explained below, success in the nearer and longer term is contingent on developing a localized strategy and capability set, well equipped to demonstrate and communicate the value rare and orphan drugs bring to patients, their caregivers and broader society.

Figure 1

Timeline of entry dates of select rare and orphan companies



*PTC Therapeutics and Aegerion have exited Japan
Source: Company websites; L.E.K. research and analysis

Figure 2

Japan rare/orphan market SWOT analysis

Strengths

- Large underlying population
- Streamlined and harmonized clinical, regulatory pathway
- Generally attractive pricing
- Market access relatively simple
- Established rare/orphan "system"
- Key opinion leaders (KOLs) receptive to new, innovative therapies
- Ecosystem of distributors supporting cell/gene therapies

Opportunities

- Continue to value innovation, drugs for orphan and rare conditions
- Existing pricing mechanisms award attractive prices to gene therapies
- Redefinition of manufacturer's role in supporting institutions with redesign of processes related to delivery
- Development of home health infrastructure will enable streamlined monitoring and follow-up
- Patients becoming more engaged in care decisions

Weaknesses

- Language barrier, cultural differences
- Interpretation of some regulatory steps for new molecules ambiguous
- No purpose-built pricing mechanism for gene therapies
- Diffuse treatment landscape
- Lack of home health infrastructure
- Limited role of manufacturers in driving change in hospital
- KOLs, advocacy bodies can be challenging counterparts

Threats

- More stringent interpretation of existing pricing rules (e.g., health technology assessments)
- Redesign of pricing system that makes gene therapies untenable
- Interpretation of regulation becomes burdensome
- Ability to effect required process change inside/outside of hospitals remains limited
- Home health infrastructure remains limited

Attractive market fundamentals at the heart of the opportunity

At the heart of industry's interest in Japan's rare and orphan market are attractive market fundamentals: a government and society long committed to providing world-class care to patients with rare and orphan diseases, a generally attractive underlying volume opportunity, low barriers to entry, preexisting infrastructure to support rapid uptake, and favorable pricing.

Specific factors to note are discussed below.

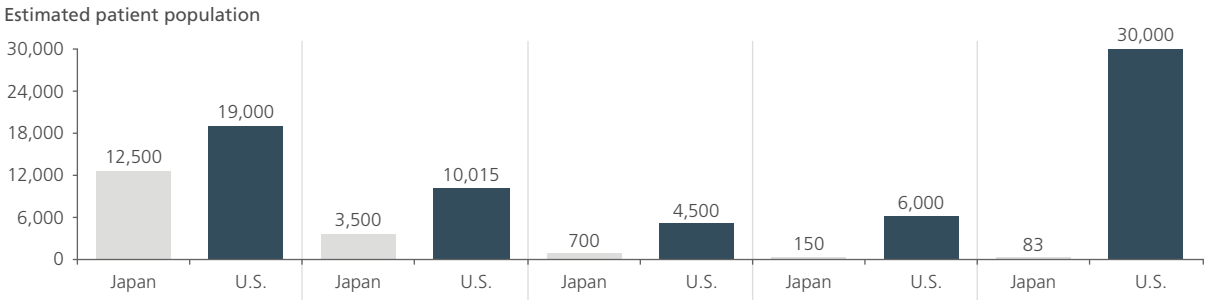
A relatively large underlying patient opportunity

Japan, despite its declining population, remains one of the largest countries in the world on a population basis. While some genetically driven rare and orphan diseases are relatively rare in Japan versus other major markets (for example, cystic fibrosis), many rare and orphan indications have reasonably comparable prevalence rates, translating to meaningful patient volume opportunities (see Figure 3).

the cost and time required for Japanese development. In rare and orphan disease treatment, this has enabled manufacturers to include very few Japanese subjects as part of global trials, increasing the practicality and efficiency of Japanese development. Orphan designation offers further benefits, including faster review times, tax benefits tied to research and development expenditure and longer market exclusivity compared to non-orphan drugs.

Furthermore, two new regulatory pathways — the regenerative medicine path and the conditional approval path — offer alternatives for companies wishing to launch as quickly as possible in Japan, although neither of these is without drawbacks. *Sakigake* Designation (for innovative products that are focused on diseases with significant need and are initially being developed for Japan) also offers compelling incentives to develop with the intent of simultaneous or earlier launch versus other major markets.

Figure 3
Estimated prevalence rates for select rare and orphan diseases



	Amyotrophic lateral sclerosis (ALS)		Duchenne muscular dystrophy (DMD)		Fabry disease (FD) — all types		Gaucher disease (GD)		Cystic fibrosis (CF)	
Prevalence rate as diagnosed (per 100,000)	10	5-7	3-5	3	0.6	1.4	0.12	1.8	0.07	40

Source: MHLW; CCF; JMDA; MDA; Novocure HP; NCBI; ALSA; NIH; NORD; NFDA; L.E.K. research and analysis

A harmonized, transparent and consultative regulatory environment

Japan's Ministry of Health, Labour and Welfare (MHLW) was a founding member of the ICH (International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use) Association and has permitted use of foreign data for regulatory submission for over a decade, dramatically reducing

MHLW is also surprisingly accessible for consultation in advance of major decisions around clinical trial design, to ensure alignment on what an approvable design would look like.

Generally favorable access and pricing

While drug pricing in general in Japan has come under pressure in recent years, in the rare and orphan market it remains relatively

favorable. L.E.K. Consulting's analysis of pricing for orphan drugs in Japan versus other major markets indicates that pricing has on average been equivalent to that in the EU3 and ~65% of that in the U.S. over the past 10 years (see Figure 4). Moreover, drugs with an orphan designation are shielded from annual price cuts. Payments to value chain intermediaries are typically low.

From the perspective of access, Japan is much simpler than other geographies — after pricing has been determined, the drug is available to all eligible patients across the country. For drugs that have indications on the intractable disease list, copayments are effectively fully subsidized by the government. For all other diseases, monthly and annual copayments are capped to ensure affordability for patients.

including Sanofi/Genzyme, Sanofi/Bioverativ and Shire/Baxalta), a pool of rare and orphan human resources exists. These individuals empathize with the rare and orphan mission, and they have experience and confidence in changing employers and are thus open to new opportunities from rare and orphan entrants.

A number of vendors — regulatory consultants, contract research organizations (CROs), recruitment consultants, specialist distributors — also exist, with a focus on and competency in supporting rare and orphan companies, including those with innovative gene and cell therapies, with their specific needs. One of the more interesting services we have become aware of is nursing services to assist rare and orphan patients with ongoing compliance, monitoring and

Figure 4
Price comparison for selected orphan drugs

Brand name	Pricing unit	Drug price (US$1.00 = 109 JPY; 1 euro = 120 JPY)			Discount rate	
		Japan	U.S.	EU3	Japan/U.S.	Japan /EU3
Spinraza (Biogen)	12 mg	9.3M JPY	16.2M JPY	13M JPY	57%	72%
Juxtapid (Aegerion)	10 mg	91K JPY	151K JPY	107K JPY	60%	85%
Rapalimus (Nobel Pharma)	1 mg	1.3K JPY	2K JPY	0.8K JPY	65%	165%
Agrylin (Shire)	0.5 mg	774 JPY	1K JPY	689 JPY	76%	112%
Romiplate (KHK*/Amgen)	250 µg	68K JPY	114K JPY	82K JPY	60%	83%
				Average	**64%**	**103%**

Note: *KHK = Kyowa Hakko Kirin
Source: MHLW; FDA; EMA; L.E.K. research and analysis

To varying extents, an established disease landscape

Many (although not all) orphan diseases, even those with few treatment options, are already well recognized in Japan, with an existing set of KOLs who are often engaged in global physician associations, standardized treatment practices, established patient advocacy bodies and patient registries — albeit of varying levels of completeness and quality. The existence of such "infrastructure" obviously streamlines entry steps.

A pool of human resources specializing in rare and orphan diseases and a broader ecosystem of specialized vendors

As a consequence of recent mergers that have created churn in the orphan and rare labor force (most recently Shire/Takeda, but also

management related to novel and challenging therapeutics for rare and orphan diseases.

Success stories have validated the opportunity for others

Success stories in the rare and orphan space abound, giving smaller companies confidence that they too can succeed in Japan. Standout examples of companies that have successfully executed on a Japan orphan and rare strategy include Alexion, Biogen, CSL Behring, Genzyme and Shire.

Validation in Japan can also create a halo effect around a given asset that signals to investors the potential value of the asset both in Japan and more broadly. A *Sakigake* Designation or eventual regulatory and marketing approval is a signal of quality, both for the asset and for the management team that has successfully navigated these hurdles.

Retaining control helps avoid poor partnering outcomes and entanglements

Beyond the obvious benefits of maximizing value capture and building a strategic beachhead, a direct presence helps entrants avoid many of the pitfalls of partnering with a local player for development and commercialization.

Specific additional considerations that make a direct strategy potentially attractive in Japan are discussed below.

Some business development teams remain unfamiliar with the rare and orphan model and struggle with appraising assets

Local business development teams often lack familiarity with the model and indicate preference for more familiar primary and specialty indications. This can lead to impasses around the relative viability of small patient volumes and the plausibility of relatively high price points. For highly innovative and disruptive therapies such as gene therapies as well as ultrarare therapies with particularly high envisioned price points, the plausibility gap may prove insurmountable.

Clinical, market access and commercial execution by inexperienced partners can go awry

Relative inexperience engaging with regulators, sites and CRO partners around clinical development programs for rare and orphan indications can pose problems for local partners. Engagement with the medical community and patient advocacy bodies can prove critical for approval, attractive pricing and access; again, local partners may have little experience with such engagement. Similarly, inexperience with the high-touch activities required for successful market preparation and patient engagement can also prove a challenge for some partners.

Risk of entanglement entailed by a local deal may complicate broader corporate goals

Further to all the above points, entering a partnership in Japan with a company that may compete with a potential suitor can add complexity to corporate-level deal-making; this complexity can of course be avoided by retaining rights and "going it alone."

The market is not without its challenges

While certain factors make the Japan market attractive, there are a number of considerations involved in direct entry. These challenges will likely be more onerous for some companies versus others and potentially weigh preponderantly on the case for direct entry.

Notable challenges to be aware of are discussed below.

Some orphan and rare diseases remain unrecognized or poorly characterized

As in other markets, some rare and orphan diseases, while present in the Japanese population, are not systematically diagnosed and are therefore difficult to address without a concerted effort to heighten awareness and build diagnostic infrastructure. This can often be the case for diseases with a relatively indolent natural history, diseases that require genetic testing for diagnosis yet lack an indicated therapy, and diseases that have social stigma attached to them (for example, some women's health disorders and some psychiatric disorders).

Pricing and access are emerging concerns

While rare and orphan pricing has historically been generally positive, the outlook appears to be less clear. There are two specific factors to heed.

The first is Health Technology Assessments (HTAs). In the pilot HTA program run by the MHLW from 2016 to 2018, rare and orphan drugs were explicitly excluded from the scope. In the formal scoping of the final HTA program, which was initiated in 2019, language was included allowing the MHLW to include drugs due to "significantly high prices," yet otherwise all indications suggested rare and orphan drugs would not be targeted (see Figure 5). However, one of the first drugs to be included in the formal HTA program was Alexion's Ultomiris (ravulizumab-cwvz), despite it being indicated for an intractable disease (paroxysmal nocturnal hemoglobinuria). During the same pricing round, Alnylam's Onpattro was awarded a price tag not dissimilar to that achieved in the U.S. yet was not considered for HTAs on the grounds that it is indicated for an intractable disease. As such, HTAs should now be anticipated in development and market access plans, even if such efforts prove to be unnecessary.

While the results from HTAs are currently intended only to adjust awarded premiums, the Ministry of Finance has made it clear that it would like HTAs, when used, to drive overall price akin to how the National Institute for Health and Care Excellence's recommendations are used in the U.K. While the MHLW is currently opposed to such an approach on the grounds this would compromise patients' access to innovation, this is an issue to track going forward.

Figure 5

Five products included in the cost-effectivenesss assessment (CEA) program since formal rollout in April 2019

Japan	Company	Category	Indication	Launch date in Japan	Peak revenue (billion JPY)
Trelegy	GSK	H1	Chronic obstructive pulmonary disease (COPD)	05/2019	23.6
Ultomiris	Alexion Pharma	H1	Paroxysmal nocturnal hemoglobinuria (PNH)	08/2019	33.1
Breztri Aerosphere	Astra Zeneca	H5	COPD	08/2019	18.9
Trintellix	Takeda Pharma	H1	Depression	11/2019	22.7
Coralan	Ono Pharma	H2	Congestive heart failure (CHF)	11/2019	5.8

Note: New products are considered for CEA if they have received Innovation, Usefulness or Improvement premiums and/or have less than 50% transparency in manufacturing cost and have forecast peak annual sales of >10 billion JPY (for H1) or 5-10 billion JPY (for H2) or have been selected by Chuikyo for evaluation (H3), typically due to significantly reimbursement prices. Products already listed before the CEA implementation follow the same eligibility and are categorized as H4. Products used as reimbursement comparators for H1-H4 products belong to H5.
Source: MHLW

Figure 6
Products eligible for CEA are assigned categories from H1 to H5

Category		Comparator method	Cost-based method	Selection criteria
(i) Newly listed products (to be listed after CEA introduction)*	H1	Premiums granted**	Premiums granted,** or cost disclosure below 50%^	Peak sales forecast: 10 billion JPY or more
	H2			Peak sales forecast: 5 to no more than 10 billion JPY
	H3			Products selected by Chuikyo due to significantly high prices, etc.^^
(ii) Already listed products (listed before CEA introduction)	H4	Premiums granted** (irrespective of pricing methods)		Sales: 100 billion JPY or more Products selected by Chuikyo due to significantly high prices, etc.^^
Similar products	H5	Products similar to H1-H4 products		Drugs for which reference drugs used in pricing are subject to CEAs; medical devices for which reference products used in pricing are subject to CEAs, and which are in the same "function" category

Note: *Even if a product's sales forecast does not meet the selection criteria upon listing, it will be selected if its annual sales exceed 5 billion JPY due to market expansions; in this case, this product will be placed into either H1 or H2 depending on the annual sales; **Products that have received premiums for innovativeness/usefulness (drugs, medical devices) and premiums for improvement (medical devices); ^For medical devices, products that do not carry the breakdown of manufacturing costs apply; ^^Products selected by Chuikyo for priority assessments include products with significantly high unit prices, and products that have already gone through the CEA process but need reexaminations due to scientific knowledge obtained after the CEAs
Source: MHLW; Pharma Japan

The second factor is the sustainability of the cost-based pricing approach. For novel therapeutics, cost-based pricing offers a path to generally attractive Japan pricing, counterintuitive as it may seem to those not familiar with Japan drug pricing. However, the level of data disclosure used to support pricing is typically sparse. The MHLW is able to claw back some price when data is not forthcoming, but many around the MHLW see the cost-based approach as subject to abuse and thus in need of revision. In the meantime, there is a strong urge to reduce the number of products priced in this manner (see Figure 6). Again, this is an issue to be tracked.

Beyond these points, there are several "moving parts" and complicating factors of which manufacturers should be aware, including pricing changes resulting from indication expansions, dose revision, potential elimination of foreign-reference pricing for drugs priced using the comparator method, and more aggressive pricing cuts to drugs that have surpassed their market exclusivity period.

KOL community can be politicized and difficult to navigate

The KOL landscape can often be "tribal," with allegiances coalescing around a handful of powerful influencers who may not be on good terms with one another. This can be compounded by the competitive overlay of clinical trials, whereby KOLs feel loyalty to a particular manufacturer with whom they partnered for clinical trials and an obligation not to cooperate with other manufacturers in the field. Some KOLs are quick to take offense and have extremely long memories. Nevertheless, KOLs are critical to the progress of many therapies through trials, regulatory approval, reimbursement and eventual commercialization and should be managed with care.

Patient advocacy bodies can be difficult to engage with productively

As in other markets, patient advocacy bodies can be invaluable for sourcing patients for trials and for advocating for regulatory and reimbursement success. However, not all bodies in Japan work productively toward this goal. There is sometimes a tendency among these bodies to regard rare and orphan manufacturers with skepticism, which can be at the expense of collaboration for the greater good of patients with the disease in question.

Some of the diagnostic and delivery infrastructure that would be taken for granted in the US/EU is absent

Specialist care in Japan is administered largely in the hospital setting. Home health service capacity for complex patients is limited. Scope of practice regulations and norms limit the care that can be provided in the absence of a physician. Meanwhile, specific regulatory and reimbursement steps are required before injectable drugs can be self-administered by the patient. These factors may complicate the opportunity for companies whose therapies are

best administered in the home setting and/or require considerable ongoing monitoring.

Diagnostics are not reimbursable unless they are intended to support a clinical decision. Penetration of next-generation sequencing (NGS) is surprisingly low, although adoption is gathering steam, driven by commercialization of targeted therapies with high demand for multiple biomarker panels and approval and reimbursement of NGS tests. As a result, manufacturers may find surprisingly limited characterization of their target indications and a great deal of work required to put in place diagnostic infrastructure and patient flows.

Pool of corporate leaders is sparse

Despite the resource pool of experienced rare and orphan human resources, those with true leadership experience, track records of success, and the ability to engage effectively with multiple stakeholder groups and to innovate new care models — as well as the linguistic and intercultural skills to work effectively with U.S.- or EU-based biopharma companies — are thin on the ground. Specialist recruitment companies, such as Morunda KK, have mapped the marketplace and can help companies find the best possible candidate list for a given search. Nevertheless, trade-offs are typically required; the perfect candidate rarely exists.

Specific considerations for gene therapies

Many gene therapy companies regard Japan as strategically core for all the reasons mentioned in the body of this piece. However, with Zolgensma's approval still stalled, there are no true precedents as to how gene therapy will play out in the Japanese context; only Kymriah provides some clues. Our current qualified view is that the opportunity is attractive, but significant areas of uncertainty, listed below, should be well understood in advance of any entry decision.

Intuition makes sense to government stakeholders

The clinical and economic benefits of a one-time "cure" for diseases that would have condemned patients to disability or death at an early age or resulted in high annual expenses over the lifetime of afflicted patients are not lost on the government. There is an appetite to bring these therapies to Japan and the patients who need them.

Medical community positive; safety concerns muted

The medical community appears largely nonplussed about gene therapy; assuming the Pharmaceuticals and Medical Devices Agency and the MHLW are satisfied with a therapy's safety profile, the physicians will largely be on board.

General comfort among patients, caregivers

Discussions with patients and advocacy bodies also suggest a general comfort with gene therapies, with the bodies unsurprisingly excited by the clinical potential of these therapies.

Emerging pricing milieu still to play out

Existing pricing mechanisms appear to allow for favorable pricing akin to that expected in other major markets, although U.S.-level pricing may be a stretch. Kymriah, for instance, received relatively high pricing. However, as mentioned in the body of the report, cost-based pricing is under scrutiny and may be redesigned. Moreover, the MHLW and other stakeholders clearly see a need for a different pricing approach for disruptive gene therapies; what that will look like is unclear.

Regulatory situation still crystallizing

A regulatory framework (e.g., around Cartagena Protocol compliance requirements) exists to guide gene therapies through development to launch. Our understanding is that these regulations are more stringent than in other markets and that review of submissions is taking longer than stated timelines would suggest.

Challenging supply chains

As in other markets, importing and moving viral vectors is no small task and requires careful planning. That being said, Japan's indigenous research base has a competency in cell therapies, and an ecosystem of logistics providers exists that can move high-value biological products with challenging storage requirements while meeting compliance requirements.

Practical challenges to institutions; can require manufacturers to reenvision care model

Institutions need to adapt existing practices and infrastructure to meet the compliance and practical requirements of gene therapies, which in turn will require support and guidance from manufacturers. Cash-flow challenges are often overlooked by manufacturers, but are very real for smaller institutions that may struggle to manage the working capital requirements of gene therapies.

Manufacturers may need to engineer new referral pathways outside of the hospital to get patients to designated sites. Within the hospital, manufacturers will likely need to work closely with stakeholders to ensure a smooth "turnkey" system is in place for the patient and product at the time of administration and to manage the patient post-discharge.

A direct presence will not make business sense for everyone

Clearly, there are a lot of considerations, both positive and negative, contributing to a Japan entry decision, and by no means will a direct entry be the optimal model for all players. A few of the broader trade-offs that should be considered — beyond those implied by the specific comments above — are noted below.

Asset-specific risk/return profile a fundamental input to decision-making

A frank assessment of the risk/return profile of opportunity is key for any entry decision. What do the economics of the envisioned opportunity and associated investments and cost base look like, and how attractive will the resulting cashflow be? What risks, measurable and otherwise, exist? How confident are we in our ability to manage these risks and successfully execute? How do the options — go it alone versus partner versus flavors in between — compare?

Broader strategic importance of Japan also matters

From a return-on-investment and a risk-diversification perspective, a direct entry will make more sense when the local subsidiary is for a pipeline of products rather than just a single asset. How does this strategic dimension influence how we think about the decision? If there is no longer-term play, manufacturers have to think very hard about whether the potential value and risk profile of a single asset can justify a direct presence.

Management bandwidth and 'comfort' are critical yet often-overlooked considerations

Many pre-commercial orphan and rare companies are simultaneously seeking to launch in the U.S. and/or EU and not only in Japan. The majority of the value of an asset and pipeline is typically driven by the U.S. opportunity, and getting the U.S. right should thus be a priority over other geographies. At the same time, a successful Japan entry requires a significant investment of management time. Management teams need to be frank about their ability to give the Japan opportunity due attention without possibly compromising success in other potentially more valuable geographies. Familiarity with and prior knowledge of Japan will of course aid the efficiency of assessing and operationalizing Japan, improve management of key risks, and give greater overall confidence in the Japan entry decision.

Checklist for prospective Japan entrants

Based on our experiences and observations, there are a number of areas around which new entrants get "stuck" that companies should keep in mind as they work through the initial business case for entry and subsequent operationalization steps. Note: This is by no means a comprehensive overview of all workstreams involved in a successful Japan market entry or launch.

- Detailed initial market assessment that critically assesses and validates key volume and pricing drivers, and risks and uncertainty around these
- Entry decision that reflects not only a rational characterization of risk and return, but also management team bandwidth
- Detailed, early understanding of the regulatory and compliance milieu, especially for highly innovative molecules, including implications for key regulatory and commercial timelines
- Early view on potential pricing method, supporting value story and engagement strategy to ensure communication of value and effective expectation setting
- Careful mapping of KOL universe and a deliberate engagement plan
- Similarly diplomatic, early engagement with patient advocacy groups to ensure a productive relationship
- Understanding of the barriers and required action steps along the patient and product flows, including which stakeholder will need to do what, to guide operationalization
- Hiring plan that indicates critical attributes of leaders for your particular asset/portfolio to inform inevitable trade-offs

Endnotes

[1] Japan Ministry of Health, Labor and Welfare, https://www.mhlw.go.jp/english/wp/wp-hw5/dl/23010223e.pdf, accessed on 12/2/2019

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