



Establishing Business in Japan

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Agenda

- Who are we?
- Why Japan?
- What is the Nanbyo-listing (Intractable Disease listing)?
- Progress so far
- How has JETRO supported us?



Who are we? Why Japan?

- Aegerion Pharmaceuticals is a small start up - a global biopharmaceutical company dedicated to the development and commercialization of innovative therapies for patients with debilitating, rare diseases.
- Preliminary country market assessment was conducted:
 - Population, HoFH estimated prevalence, HoFH estimated market size, estimated reimbursement, and market potential
 - Criteria was also assessed in comparison with other countries (i.e. market size, political environment, economic risk, biotech/pharmaceutical environment, investment required and regulatory timelines/requirements (i.e. local clinical study)
 - Considerations were also assessed around the rare disease space and orphan drug designation benefits
 - Homozygous Familial hypercholesterolemia (HoFH) is one of the 56 Intractable Diseases listed in the National Program on Rare and Intractable Diseases (NANBYO)



Nanbyo (Intractable Disease)

“Nanbyo” Definition (from 1972)

1. Diseases that have resulted from an unidentifiable cause and, without a clearly established treatment, have a considerably high risk of disability
2. Diseases that chronically develop and require a significant amount of labor for the patient’s care, causing a heavy burden on other family members of the patient, both financially and mentally.

Tokutei Shikkan (Specified Rare and Intractable Diseases)

- This refers to the type of rare, intractable disease that has resulted from an unidentifiable cause and is very difficult to treat without an established treatment procedure. This type of intractable disease develops chronically and may have serious consequences that make it very difficult or even impossible for patients to return to school or work. Treatment of these designated diseases is very expensive, carrying a high cost of long-term care and medicine, which causes great financial and mental stress on the patient’s family.
- Sources: <http://www.nanbyou.or.jp/English/index.htm>
- <http://www.nanbyou.or.jp/entry/4141>



Tokutei Shikkan: Specific Rare & Intractable List

- 56 diseases were listed up until end of 2014
- 110 diseases were listed in January 2015
- The list increased to 306 diseases in May 2015
- All subsidized from public expense

- **Budget:**
 - March 2012 (Actual):
 - 40 Billion Yen
 - # of patients: 780,000
 - # of specified rare and intractable diseases= 56

 - March 2016 (Projection):
 - 182 Billion Yen
 - # of patients: 1,500,000
 - # of specified rare and intractable diseases=306

Japan Intractable Diseases Information Center

What is an intractable disease?

- [Definition of Nanbyo \(Intractable Diseases\)](#)
- [The Specified Disease Treatment Research Program](#)
- [Disease list for Specified Disease Treatment Research Program](#)

The Specified Disease Treatment Research Program ("Tokutei Shikkan Chiryō Kenkyū Jigyō")

The Specified Disease Treatment Research Program subsidizes medical care for patients of rare and intractable diseases. Although thirty percent co-payment is required for insurance-covered medical care in most cases, the national government and prefectures partly cover the patients' share of medical expenses by this program. Currently, this system applies to 56 intractable diseases.

[Disease list for Specified Disease Treatment Research Program](#)

Each one of these diseases has a criterion for designation. Based on a doctor's diagnosis, a patient must file an application to the government of the prefecture he or she lives in. If the application is accepted, a Certificate of a Recipient of Designated Disease Treatment is issued. Refer to the guidelines for the system and related procedures.



Agerion Pharmaceuticals K.K.

- **September 2013 Milestone:**
 - Legal entity established
 - Orphan drug designation (ODD) granted for Lomitapide
- **2015 Milestone:**
 - Japan Phase III study conducted
- **January 2016 Milestone:**
 - Japanese NDA for Lomitapide submitted
- **Focus:**
 - HoFH (Homozygous Familial Hypercholesterolemia), a rare genetic lipid disorder (Intractable disease list)



GQP

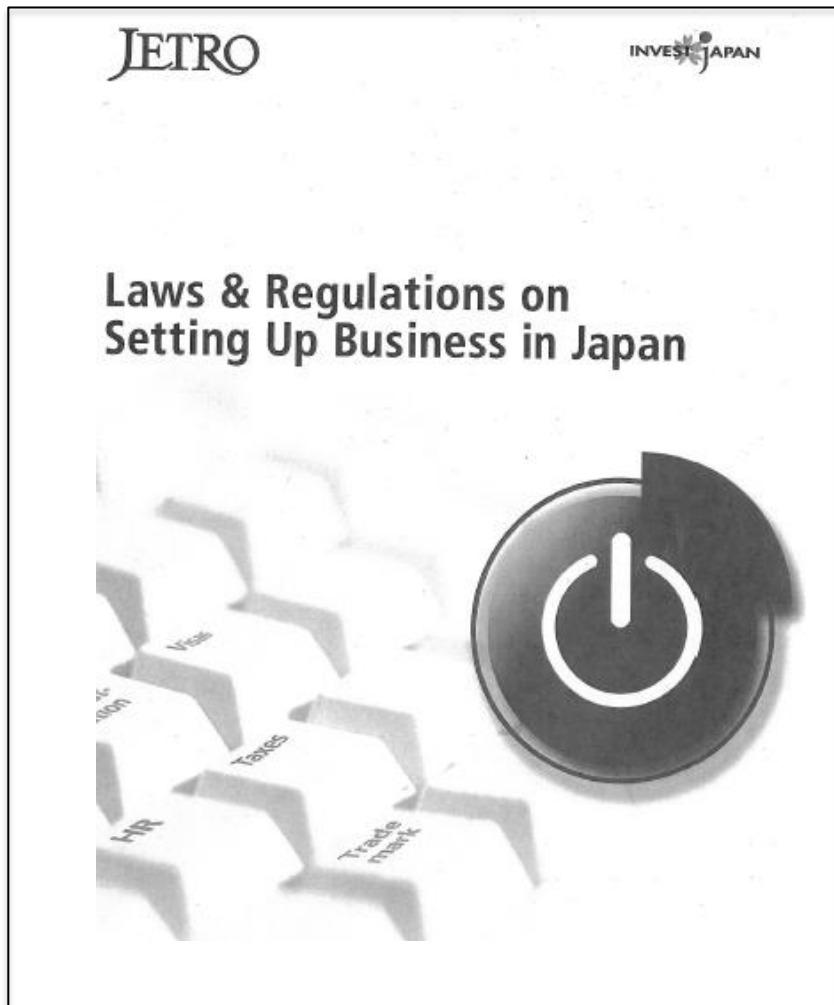
MSG

GVP

Opened office with MAH
required resources



JETRO Resources



Advisors & Consultants:

- Law
- Regulatory
- Finance
- Orphan Drug
- Tax
- Human Resources



Together...明るい未来に向けて！！！！

Towards a Bright Future...

Ark Hills, Ark Mori, Akasaka, Tokyo



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