COMPANY RESEARCH AND ANALYSIS REPORT

SymBio Pharmaceuticals Limited

4582

JASDAQ Growth Market

20-Apr.-2020

FISCO Ltd. Analyst
Yuzuru Sato

FISCO Ltd.
http://www.fisco.co.jp

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https://www.symbiopharma.com/ir_e/

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Summary

Seeking to become a global specialty pharmaceutical company

Symbio Pharmaceuticals Limited <4582> (hereafter, also "the Company") is a bio-venture that is advancing developments in the fields of oncology, hematology, and rare diseases where there are few patients, but high medical needs. The main drugs in the development pipeline are TREAKISYM®, whose indications as a treatment for malignant lymphoma are expanding, and rigosertib, which is being developed for myelodysplastic syndrome (MDS). In September 2019, the antiviral drug brincidofovir (hereafter, "BCV") was added to the development pipeline. The Company entered into a global licensing agreement for BCV, and the Company will develop the drug not only in Japan but overseas as well, as the Company seeks to become a global specialty pharmaceutical company.

1. Steady progress being made for becoming profitable in FY12/21

The Company has three key strategies to become profitable in FY12/21: building its own sales platform for TREAKISYM®, launching sales of a high-added-value liquid formulation (ready-to-dilute) ("RTD") for TREAKISYM®, and expanding the indications for TREAKISYM®. Steady progress is being made on all three strategies. First, the Company's marketing agreement with Eisai Co., Ltd. <4523> for TREAKISYM® expires in December 2020, and the Company is building a system that will allow it to quickly conduct sales after the agreement expires. By the end of Q2 FY12/20, the Company will complete building its own supply chain function, including sales representatives along with a distribution and logistics system. Second, the Company submitted an NDA for TREAKISYM® RTD liquid formulation in September 2019, and expects to commence sales of the RTD liquid formulation in Q1 FY12/21 if everything proceeds smoothly, and to switch from the existing lyophilized powder formulation type in one year. Building its own sales platform and switching to the RTD formulation will significantly improve the profitability of TREAKISYM®. In terms of expanded indications for TREAKISYM®, the Company plans to submit an application for relapsed and refractory diffuse large B-cell lymphoma (DLBCL) in Q2 FY12/20, and by launching sales in approximately Q3 FY12/21, sales of TREAKISYM® will likely increase further due to the growth in the number of patients receiving the drug. If these strategies progress smoothly, the Company expects net sales of ¥9,008mn and operating profit of ¥1,031mn in FY12/21.

2. Trends in the other development pipeline

For rigosertib (intravenous formulation), U.S. licensor Onconova Therapeutics, Inc. ("Onconova" "ONTX") is conducting a global Phase III clinical trial, and top-line results (primary endpoints) are expected to be released in H2 FY2020. Based on the results of the trial, the Company is planning to apply for approval in Japan at the same time as in the U.S. and Europe, and is aiming to commence sales in 2023. Also, BCV, incensed-in in September 2019 from Chimerix Inc. ("Chimerix") (U.S.), stands out for its ability to drastically suppress the proliferation of DNA viruses and for its superior safety profile. BCV is expected to be developed into an effective treatment against a wide spectrum of infectious diseases caused by DNA viruses. As the first step, the Company aims to begin clinical trials in Japan targeting viral hemorrhagic cystitis (vHC)* occurring after allogeneic hematopoietic stem cell transplantation in H2 FY12/20. In addition, the Company plans to pursue business roll-out overseas, including a possible partnership strategy, that takes the regional characteristics of the targeted diseases into consideration. BCV is expected to be effective for treating viral infectious diseases following organ transplants, and the Company's target markets include Europe, the U.S, and the Asian region including China as key markets, so this drug has tremendous potential. The Company will decide its development strategy in overseas markets by Q3 FY12/20. The Company has acquired manufacturing rights for BCV, and is currently in the process of selecting a manufacturing contractor ahead of clinical trials.

> * Viral hemorrhagic cystitis (vHC): vHC is one type of viral infectious disease that frequently occurs following hematopoietic stem cell transplantation. In vHC, hemorrhagic cystitis is caused by the proliferation of BK virus or adenovirus. In Japan, the incidence ratio of vHC for allogeneic stem cell transplantation is between 8.6% and 24.0%. There are also reports that the incidence ratio of vHC for cord blood stem cell transplantation is even higher. It is generally refractory, with patients showing primary symptoms such as frequent urination, abdominal pain, micturition pain and hematuria. In mild cases of vHC, patients often experience no symptoms. However, in severe cases, disseminated infection can be lethal. There are also reported cases where mortality occurs from renal impairment associated with adenovirus infection. Transplantation with unrelated donors including cord blood, of which there is a high proportion in Japan, is a potent risk factor. Since there are no approved drug therapies or definitive treatment in Japan, some physicians privately import cidofovir (CDV) and administer it to their patients. However, CDV has a strong nephrotoxicity with only limited efficacy, so an effective and safe drug therapy is eagerly awaited.

3. Results trends

In terms of results for FY12/19, the Company reported net sales of ¥2,837mn, down 26.0% year-on-year (YoY) and an operating loss of ¥4,301mn (compared with a loss of ¥2,656mn in the previous year). The main reason for the decrease in net sales was that sales of TREAKISYM® were halted temporarily due to quality issues at the supplier. On the cost front, selling, general and administrative (SG&A) expenses rose 34.9% YoY to ¥5,166mn due to the booking of upfront payments for the licensing of BCV and costs associated with building the Company's own sales platform. This in turn led to the larger operating loss. For FY12/20, the Company is projecting net sales of ¥3,404mn (+20.0 YoY) and an operating loss of ¥5,090mn. Although a recovery in net sales is expected due to the calming down of the quality issue, both costs associated with building its own sales platform and research and development (R&D) expenses are expected to increase, so the Company is forecasting that an operating loss will continue. The Company initiated arbitration against The Medicines Company in the U.S. with the International Chamber of Commerce (ICC), seeking damages of $82mn based on The Medicines Company's repudiation of the licensing agreement. The outcome of the arbitration is expected to be announced between March and June 2020, but this is not factored into the forecasted results.

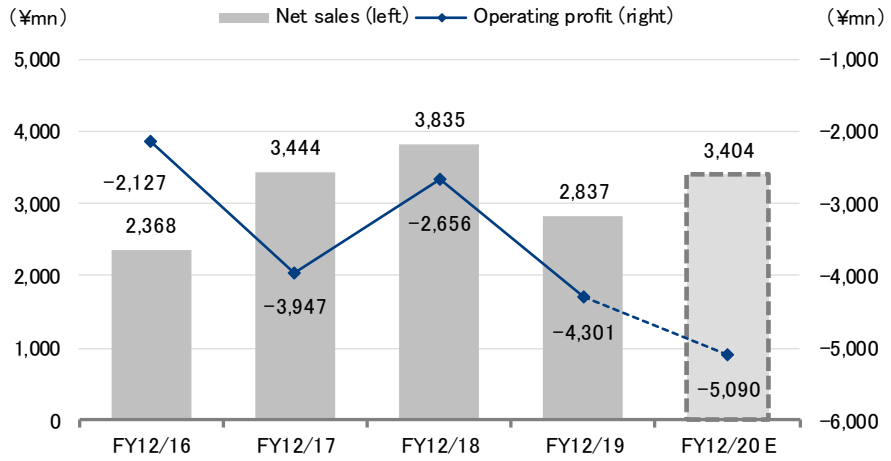
4. Mid-Range Plan

As performance targets in its Mid-Range Plan, for FY12/22 the Company has set a net sales target of ¥10,816mn and an operating profit target of ¥1,482mn, as the Company has set a target of sustainably securing an operating profit margin of at least 10%. In order to achieve these targets, in addition to becoming profitable in FY12/21, the Company plans to work on business development for BCV both in Japan and overseas, and on in-house manufacturing of BCV in order to realize high profitability and high quality.

Key Points

- In FY12/19, net sales declined due to quality issues with TREAKISYM®, and both R&D expenses and costs to build the Company's own sales system increased
- Targeting a turn to profitability in FY12/21 and continuous operating profit margin of at least 10%.
- Sales growth potential will further increase if global development of BCV progresses

Results trends



Source: Prepared by FISCO from the Company's financial results

Company profile

A bio-venture that conducts developments from the clinical-trials stage, targeting the fields of oncology, hematology, and rare diseases

1. History

SymBio Pharmaceuticals is a bio-venture founded by the current Representative Director and President Chief Executive Officer Fuminori Yoshida in March 2005. For its business strategy, its basic policy is to conduct drug discovery and development for Underserved Therapeutic Areas in which development has not been progressed due to the small numbers of patients. One of its features has a business model that aims to achieve highly efficient and rapid drug discovery within the areas targeting oncology, hematology, and rare diseases, which are fields with high medical needs, by licensing-in development candidates for which POC* for humans has been obtained, and conducting development from the clinical trials stage.

* POC (Proof of Concept): when the usefulness and efficacy of a new drug candidate compound is recognized following its administration to animals or humans during research and development.

The development candidate licensed-in first was the anti-cancer agent bendamustine hydrochloride (hereafter, Bendamustine hydrochloride; product name in Japan, TREAKISYM®) indicated for malignant lymphoma that was developed by Astellas Pharma GmbH (Germany), for which the Company concluded an exclusive development and marketing rights agreement for Japan in December 2005. With the development code SyB L-0501, the Company began the phase I clinical trial in 2006 for indications for relapsed and refractory low-grade non-Hodgkin's lymphoma (NHL) and mantle cell lymphoma (MCL)*, and in 2010, it acquired manufacturing and marketing approval. It progressed licensing activities during this time, and in 2007, it expanded the target areas for the exclusive development and marketing rights in China, South Korea, Taiwan, and Singapore. Then with Eisai as its marketing partner, it concluded licensing agreements for Japan in 2008 and for South Korea and Singapore in 2009. Due to Eisai's change in business strategy, the Company has decided to dissolve the licensing agreements with Eisai on December 9, 2020. For this reason, it plans to shift to its own sales system in Japan from 2021 onward. At present, the Company is making preparations to establish sales and distribution systems.

TREAKISYM®, whose sales were launched in Japan in December 2010, continued to be subsequently developed in order to expand its indications, and in 2016, it acquired approval for indications for chronic lymphocytic leukemia(CLL) and untreated (first line of treatment) low-grade NHL/MCL, and its sales are growing. Also, in Asia, its sales began in Singapore in 2010, in South Korea in 2011, and in Taiwan in 2012. For Taiwan, in 2008, the Company concluded a licensing agreement with InnoPharmax Inc. (Taiwan), which is conducting sales through the Company.

Also, the second drug licensed-in was rigosertib (development code, SyB L-1101 (intravenous formulation)/ SyB C-1101 (oral formulation)), which is a development candidate from Onconova Therapeutics, Inc. (hereafter, Onconova) indicated for myelodysplastic syndrome (MDS)*1 for which the Company concluded an exclusive development and marketing rights agreement in 2011 for Japan and South Korea. Currently also, its development is being progressed. Further, in 2017 it concluded an exclusive development and marketing rights agreement for Japan with Eagle Pharmaceuticals, Inc. <EGRX> (U.S.) for the TREAKISYM® liquid formulation, ready-to-dilute (RTD) formulation / rapid infusion (RI) formulation (development code, SyB L-1701/SyB L-1702)*2, and in the same way, its development is being progressed.

*1 MDS is a disease in which the patient cannot produce normal blood cells due to abnormalities in the hematopoietic stem cells in the bone marrow, causing a decrease in normal blood cells and symptoms such as anemia, infection and hemorrhage. It is also known to transition to become acute myeloid leukemia. The condition of the bone marrow is examined, the leukemia transition period is determined, and it is classified into four stages, such as according to the length of the period. The high-risk type has a 25% leukemia transition period of 0.2 of a year, and the 50% survival period median value is 0.4 of a year. There are approximately 11,000 patients in Japan. The only treatment of the root cause is hematopoietic stem cell transplantation. In chemical therapy, azacitidine is used as the drug of first choice. In Japan, Vidaza® of Nippon Shinyaku Co., Ltd.'s Vidaza® <4516> is on the market, and it has annual sales on a scale of ¥15 to ¥16bn on a drug-price basis.

*2 Currently, TREAKISYM®, which has been approved in Japan, is a lyophilized powder formulation, which means it must be dissolved at the medical site when it is used. As this task is unnecessary for the liquid formulation, it greatly reduces the workload placed on healthcare workers. Also, the difference between the RTD formulation and the RI formulation is the intravenous injection time. The RTD formulation takes the same time, 60 minutes, as existing products, but the time for the RI formulation is as short as 10 minutes, so the burden on the patient is greatly reduced.

Moreover, in September 2019, the Company concluded an exclusive global license agreement with Chimerix that gives the Company the rights to develop, manufacture, and commercialize BCV in all viral diseases excluding small pox. BCV will be the third drug to be licensed-in and developed by the Company. The main features of BCV are that it has higher anti-viral activity and a superior safety profile in comparison with cidofovir (CDV: unapproved in Japan). Accordingly, BCV is expected to be an effective treatment against various infection diseases caused by DNA viruses. As the first step, the Company plans to conduct development in Japan for the indications of viral hemorrhagic cystitis (vHC), conditions that can occur after allogeneic hematopoietic stem cell transplantation.

Technology licensing-in agreements

Name	TREAKISYM®			Rigosertib sodium	Brincidofovir
Development code	SyB L-0501 (Lyophilized powder formulation) SyB C-0501 (Oral formulation)	SyB L-0501 (Lyophilized powder formulation) SyB C-0501 (Oral formulation)	SyB L-1701 (RTD formulation) SyB L-1702 (RI formulation)	SyB L-1101 (Intravenous formulation) SyB C-1101 (Oral formulation)	SyB V-1901 (Intravenous formulation)
Licensing-in partner	Astellas Pharma (Germany)	Astellas Deutschland (Germany)	Eagle Pharmaceuticals, Inc. (U.S.)	Onconova Therapeutics, Inc. (U.S.)	Chimerix Inc. (U.S.)
Date agreement was concluded / agreement period	December 2005 / Whichever is longer; the 10-year period from the first product sales, or the market-exclusive period in Japan	March 2007 / Whichever longer; the 10-year period from the first product sales or the market-exclusive period	September 2017 / Whichever longer; the product-patent period or the market-exclusive period	July 2011 / Whichever longer; the 10-year period from the first product sales (7 years in South Korea), the market-exclusive period, or the patent-validity period, in each country	September 2019
Content of the main agreements	Exclusive development and marketing rights in Japan	Exclusive development rights and marketing rights in China (including Hong Kong), Taiwan, South Korea, and Singapore	Exclusive development rights and marketing rights in Japan	Exclusive development rights and marketing rights in Japan and South Korea	Exclusive global license agreement concerning the rights to develop, manufacture, and commercialize BCV in all DNA virus indications excluding smallpox

Source: Prepared by FISCO from the Company's securities report and news release

Technology licensing-out agreements

	SyB L-0501 (Lyophilized powder formulation)			
Licensing-out partner	InnoPharmax Inc. (Taiwan)	Eisai Co., Ltd. (Japan)		Cephalon, Inc. (U.S.)
Date agreement was concluded / agreement period	March 2008 / 10 years from the first product sales in Taiwan	From August 2008 to December 2020	From May 2009 to December 2020	March 2009 / 10 years from the first product sales in China
Content of the main agreements	Exclusive development rights and marketing rights in Taiwan	Joint development rights and exclusive marketing rights in Japan	Exclusive development rights and marketing rights in South Korea and Singapore	Exclusive development rights and marketing rights in China (including Hong Kong)

Source: Prepared by FISCO from the Company's securities report

History

Date	Summary
March 2005	Established SymBio Pharmaceuticals Limited at Minato-ku, Tokyo
December 2005	Concluded a license agreement with Astellas Pharma GmbH (Germany) to acquire exclusive development and marketing rights in Japan for anti-cancer agent Bendamustine Hydrochloride
March 2006	Obtained manufacturer's license (packaging, labeling and storage) from Tokyo Metropolitan Government
March 2007	Concluded a license agreement with Astellas Deutschland GmbH (Germany) to acquire development and marketing rights in China, Taiwan, South Korea and Singapore for anti-cancer agent SyB L-0501
August 2008	Concluded a license agreement with Eisai Co., Ltd. to grant co-development and marketing rights in Japan for anti-cancer agent SyB L-0501
March 2009	Concluded sublicense agreement with Cephalon, Inc. (U.S.) to grant development and marketing rights in China for anti-cancer agent SyB L-0501
May 2009	Concluded a license agreement with Eisai to grant co-development and marketing rights in South Korea and Singapore for anti-cancer agent SyB L-0501
September 2010	Launched SYMBENDA® (generic name: bendamustine hydrochloride) in Singapore for the treatment of low-grade non-Hodgkin's lymphoma and chronic lymphocytic leukemia
October 2010	Acquired manufacturing and marketing approval of anti-cancer agent for malignant lymphoma TREAKISYM® in Japan (launched in December 2010)
July 2011	Concluded a license agreement with Onconova Therapeutics, Inc. for anti-cancer agents SyB L-1101/SyB C-1101
October 2011	Launched SYMBENDA® (generic name: bendamustine hydrochloride) in South Korea for the treatment of chronic lymphocytic leukemia and multiple myeloma
October 2011	Listed on Osaka Securities Exchange JASDAQ Growth Market
February 2012	Launched INNOMUSTINE® in Taiwan for the treatment of low-grade non-Hodgkin's lymphoma and chronic lymphocytic leukemia
October 2015	Concluded a licensing agreement with The Medicines Company (U.S.) to acquire exclusive development and marketing rights in Japan for post-operative, self-administered pain-management medication, SyB P-1501 (the agreement ended in November 2017)
May 2016	Established SymBio Pharma USA, Inc. at Menlo Park, California, USA
August 2016	Acquired manufacturing and marketing approval of anti-cancer agent for malignant lymphoma TREAKISYM® in Japan for the additional indication of chronic lymphocytic leukemia
December 2016	Acquired manufacturing and marketing approval of anti-cancer agent for malignant lymphoma TREAKISYM® in Japan for the additional indication of first-line treatment of low-grade non-Hodgkin's lymphoma and mantle cell lymphoma
September 2017	Concluded a license agreement with Eagle Pharmaceuticals, Inc. to acquire development and marketing rights in Japan for bendamustine liquid formulations (RTD formulation and RI formulation) *RTD: Ready-to-dilute, RI: Rapid Infusion
October 2017	Filed for arbitration for damages against The Medicines Company (U.S.) due to the non-fulfillment of the licensing agreement
July 2018	TREAKISYM® was newly listed as the standard treatment for malignant lymphoma in the 2018 edition of the Japan Society of Hematology's Guidelines for the Treatment of Hematopoietic Tumors,
September 2019	Concluded an exclusive global license agreement with Chimerix (U.S.) concerning the rights to develop, manufacture, and commercialize the antiviral drug, brincidofovir (excluding smallpox)

Source: Prepared by FISCO from the Company's securities report and website

Profit margin is expected to increase with the expansion of indications for TREAKISYM® as the standard treatment for malignant lymphoma and progress in the shift to the RTD liquid formulation from 2021 onward

2. Trends in the development pipeline

(1) TREAKISYM® (generic name: bendamustine hydrochloride)

TREAKISYM® is an anti-cancer agent for malignant lymphoma. Malignant lymphoma is a disease in which lymphocytes, which are a type of white blood cell, undergo canceration (tumorification) and lumps (masses) can grow in lymph nodes and organs other than lymph nodes (such as the stomach, intestines, thyroid, spinal cord, lung, liver, skin, and eyes) distributed throughout the body. It is said to be the most common of the blood cancers, with approximately 10 out of every 100,000 people contracting it each year in Japan. Malignant lymphoma is mainly divided into Hodgkin's lymphoma (HL) and non-Hodgkin's lymphoma (NHL), with about 90% of cases in Japan being NHL. It is classified into low-grade, medium-grade, and high-grade according to the progression rate of the symptoms, and there are various disease types.

Types of non-Hodgkin's lymphoma

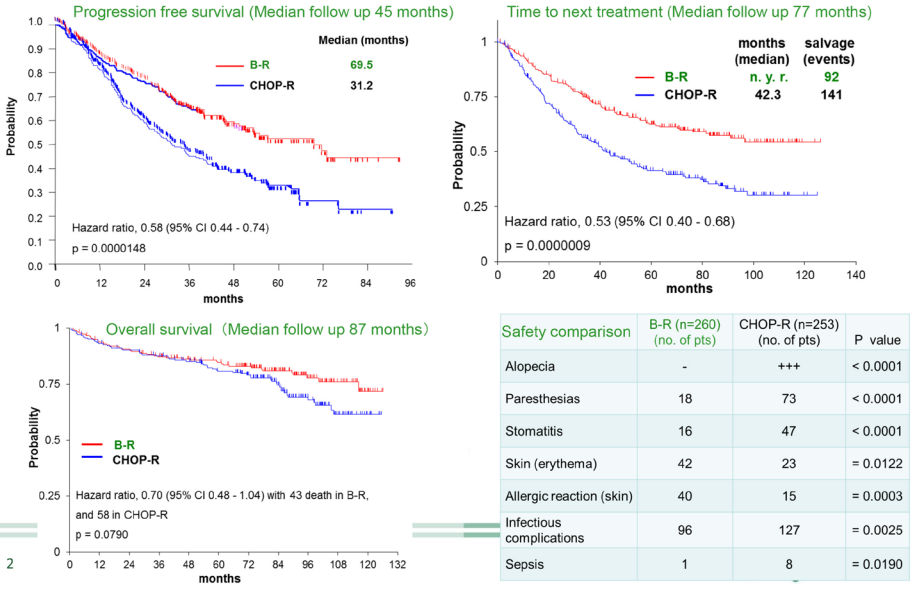
Type according to grade	Non-Hodgkin's lymphoma type (disease type)
Low grade: Indolent lymphoma (progresses yearly)	Follicular lymphoma (grade 1, 2), MALT lymphoma, lymphoplasmacytic lymphoma Mycosis fungoides, Sezary syndrome, chronic lymphocytic leukemia / small lymphocytic lymphoma, etc.
Medium grade: Aggressive lymphoma (progresses monthly)	Follicular lymphoma (grade 3), mantle cell lymphoma, diffuse large B-cell lymphoma Peripheral T cell lymphoma, extranodal NK / T cell lymphoma, adult T cell leukemia / lymphoma (chronic type), etc.
High grade: Highly aggressive lymphoma (progresses weekly)	Burkitt's lymphoma, acute lymphocytic leukemia / lymphoblastic lymphoma Adult T-cell leukemia / lymphoma (acute type, lymphoma type), etc.

Source: Prepared by FISCO from National Cancer Center Hospital materials

Among these, currently the Company has acquired marketing approval for indications for relapsed and refractory low-grade non-Hodgkin's lymphoma (NHL), mantle cell lymphoma (MCL), chronic lymphocytic leukemia (CLL), and untreated (first line of treatment) low-grade NHL/MCL. In particular, in 2016 its use in this field started to spread following the acquisition of marketing approval for untreated low-grade NHL/MCL, and then in July 2018, in its treatment guidelines, the Japan Society of Hematology (JSH) recommended TREAKISYM® and Rituximab® combination therapy (BR therapy) as the standard treatment, and it is becoming established as the standard treatment in both name and reality. In the field of untreated (first line of treatment) low-grade NHL, previously the standard treatment was R-CHOP therapy*, but on looking at the market penetration rates, after Q4 FY12/17 (October to December 2017), BR therapy had overtaken it, and as of Q2 FY12/19 (April to June), BR therapy had a 55% share of the market as a whole. In light of the high efficacy of BR therapy, the Company aims to increase the market penetration rate up to 64% in 2020 in the untreated area at the end of 2020.

* R-CHOP therapy: a multi-drug combination therapy combining Rituximab® and 4 other drugs

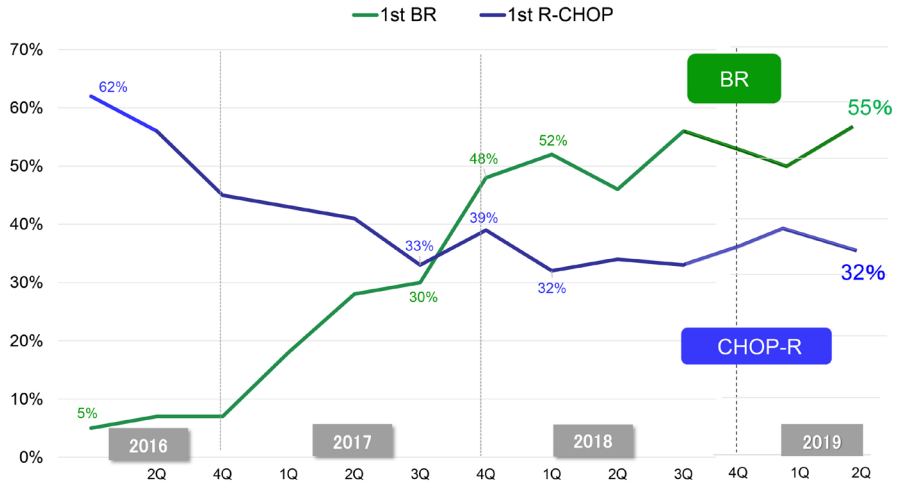
BR therapy It is superior to R-CHOP for both efficacy and safety



Safety comparison	B-R (n=260) (no. of pts)	CHOP-R (n=253) (no. of pts)	P value
Alopecia	-	+++	< 0.0001
Paresthesias	18	73	< 0.0001
Stomatitis	16	47	< 0.0001
Skin (erythema)	42	23	= 0.0122
Allergic reaction (skin)	40	15	= 0.0003
Infectious complications	96	127	= 0.0025
Sepsis	1	8	= 0.0190

Source: From the Company's results briefing material

Treatments for untreated (first line of treatment), low-grade NHL patients



Source: From the Company's results briefing material

The Company is currently progressing five drugs in the development pipeline. Of these, in the phase III clinical trial for the expansion of the indication of the existing lyophilized powder formulation TREAKISYM® for relapsed and refractory DLBCL, the observation periods of all test subjects were completed in September 2019. On November 5, 2019, the Company announced favorable results, with the overall response rate, which was the primary endpoint of the trial, exceeding the expected response rate. Currently, the Company is preparing to submit a new drug application (NDA) during Q2 FY12/20. If steady progress is made, it is expected that approval could be granted and sales commence in Q3 FY12/21. If relapsed and refractory DLBCL is approved as an indication, the potential market size for TREAKISYM® will grow to more than double the size of the existing market. This is because the number of patients for all three areas covered by existing indications is currently a little less than 10,000 but the number of relapsed and refractory DLBCL patients will increase by 1.5 times if the new indication is approved. Patient advocacy groups and relevant academic societies have also filed petitions urging the authorities to make BR therapy available as early as possible. As soon as sales begin, TREAKISYM® is expected to rapidly penetrate the market in the field of relapsed and refractory DLBCL.

In September 2019, the Company applied for marketing approval for the RTD formulation, which is the TREAKISYM® liquid formulation. If steady progress is made, the Company expects to obtain approval in Q4 FY12/20 and to launch sales in Q1 FY12/21. The Company plans to switch 95% of the product from the current lyophilized powder formulation to the RTD formulation by the end of 2021, and from the beginning of 2022 the Company will aim to quickly achieve a 100% switch. Also, the Company started clinical trials of the RI formulation in November 2018 with the main aim of confirming its safety (planned number of cases, 36), and is aiming to complete clinical trials in H1 FY12/20, and launch sales in H1 FY12/22. The indications for the RTD/RI formulations include all those for which TREAKISYM® has already been approved as well as relapsed and refractory DLBCL.

Teva Pharmaceuctical Industries Ltd. (U.S.) has already commercialized the RTD/RI formulations as BENDEKA® on the U.S. market. With the acquisition of a 97% share of the bendamustine market as of 2017, it appears that most patients have already switched to the liquid formulation. In addition to reducing the burden on healthcare professionals by eliminating the need to dissolve the drug, the administration time for the RI formulation is only 10 minutes (compared to 60 minutes for the existing products and RTD formulation), so the burden on patients is also greatly reduced. Therefore, there are strong calls for its early marketing approval in Japan. As the exclusive sales period for the existing, lyophilized powder formulation type in Japan ends in 2020, generics may be developed for it. But if the RTD/RI formulations are launched, there will be major differences in terms of their functions, so this would effectively extend the exclusive marketing period until 2031. If the RTD/RI formulations are launched, the drug prices will be the same level as the previous products, but the supplier will be changed to Eagle Pharmaceuticals, so at FISCO we think it is highly likely that the profit margin will improve compared to the existing products.

For the treatment of malignant lymphoma, TREAKISYM® obtained approval for use as a pretreatment agent for the chimeric antigen receptor T-cell (CAR-T) therapy (KYMRIAH® intravenous transfusion), which obtained NHI price listing in May 2019. TREAKISYM® is also currently used as a drug in combination therapy in multiple clinical trials. Most recently, in February 2020, it was announced that the primary endpoints have been achieved in a domestic Phase II clinical trial (Polatuzumab vedotin + BR combination therapy) being conducted by Chugai Pharmaceutical Co., Ltd. <4519> to treat relapsed and refractory DLBCL patients. This therapy received marketing approval in the U.S. in June 2019 and in Europe in January 2020, so there is a high likelihood that it will receive approval in Japan as well. Whether to treat relapsed and refractory DLBCL patients with BR therapy or Polatuzumab vedotin + BR therapy will be determined by doctors based on a patient's condition, but in both cases TREAKISYM® will be used. These developments signal that TREAKISYM® now has an even more solid position as a standard treatment for the future, and at FISCO we believe that it will result in an expansion of the market for TREAKISYM®.

* Marketed by Novartis Pharma (Switzerland). The areas of indication are CD19 positive recurrent/refractory B-cell acute lymphoblastic leukemia and DLBCL (there are multiple other requirements for administration), and in Japan it is believed that there are just under 250 patients per year who could be administered this drug.

The Company has decided to abort development of TREAKISYM® oral formulation (development code: SyB C-0501) for progressive solid tumors and systemic lupus erythematosus (SLE). This decision was made because the results of trials did not exceed expectations, and, in order to best utilize limited management resources, the Company made the decision to prioritize the domestic and overseas development of BCV, for which the Company has newly acquired a license as a part of its business strategy.

* An autoimmune disease in which the patient's own immune system mistakenly attacks normal cells. It is designated as an intractable disease because it causes inflammation and tissue damage in various organs throughout the body. There are approximately 60,000 to 100,000 patients in Japan.

TREAKISYM®

Drug	Indication	Progress
SyB L-0501(FD lyophilized powder formulation)	r/r low Low-grade NHL/MCL	Approved October 2010
	CLL	Approved August 2016
	1st line Low-grade NHL/MCL	Approved December 2016
	r/r DLBCL	P3 completed and achieved the primary endpoint, scheduled to apply for marketing approval in Q2 FY12/20
SyB L-1701 (RTD liquid formulation)	All indications	Applied for marketing approval in September 2019, targeting launch in Q1 FY12/21
SyB L-1702 (RI liquid formulation)	All indications	Currently undergoing clinical trials, complete trials in the first half of FY12/20, targeting launch in the first half of FY12/22

Source: Prepared by FISCO from the Company's results briefing material and website

(2) Rigosertib (intravenous formulation/oral formulation)

Rigosertib is an anti-cancer agent that has a unique multi-kinase inhibitory action (which causes cancer cells to die by inhibiting the multiple kinases involved in cancer cell proliferation, invasion and metastasis). Its development is being progressed indicated for high-risk myelodysplastic syndrome.

For the current development situation, a global Phase III clinical trial is being conducted by licensor Onconova for the intravenous formulation indicated for relapsed and refractory high-risk MDS (final target 360 patients), and as of November 2019 more than 90% of the target number of patients had been registered. In Japan, the Company's assigned area, the Company has registered subjects for 48 of the target number of 50 cases as of the end of December 2019. In H1 FY12/20, Onconova plans to announce the top-line results (primary endpoints). Based on the results of the trial, the Company is planning to apply for approval in Japan at the same time as in Europe and the U.S. The aim is to submit the application for marketing approval in 2021, and receive approval in Q4 FY12/22. The potential market size is seen as being roughly the same as Vidaza®, at ¥15 to ¥16bn.

For the oral formulation, the Phase I clinical trial was completed for the single drug indicated for relapsed and refractory high-risk MDS in June 2019, while in the future, the plan is to switch to development for its joint use with azacitidine. However, it appears that development is now being given lower priority for the time being, as Onconova's business strategy is to first aim for marketing approval for an intravenous formulation for high-risk MDS, and also because a physician-led phase I trial targeting progressive KRAS* positive NSCLC (non-small cell lung cancer) as a new indication is going to start in 2020. Onconova has confirmed a tumor suppressive effect in a KRAS-positive non-small cell carcinoma animal model by using an immune checkpoint inhibitor and rigosertib in combination, and is expected to proceed with clinical development.

* KRAS is a cancer gene. The KRAS gene usually inhibits the replication of cells, but when a mutation of the KRAS gene occurs, a signal for cells to replicate is continually sent out, which causes cancerous cells to actively replicate. Patients with mutated KRAS genes have been identified in a considerable percentage of patients with certain forms of cancer, including colorectal cancer and lung cancer.

Rigosertib

Drug	Indication	Progress
SyB L-1101(Intravenous formulation)	Relapsed and refractory high-risk MDS	P3 global clinical trials ongoing Onconova plans to announce top-line data in H1 FY2020 If satisfactory, plans to apply for approval in Japan at the same time as in the U.S. and Europe
SyB C-1101 (Oral formulation)	Relapsed and refractory high-risk MDS single drug	P1 completed
	Untreated high-risk MDS (AZA combination use)	Under preparation
	Untreated high-risk MDS (AZA combination use)	P2/3 global clinical trial under preparation at Onconova If it starts, the Company plans to participate

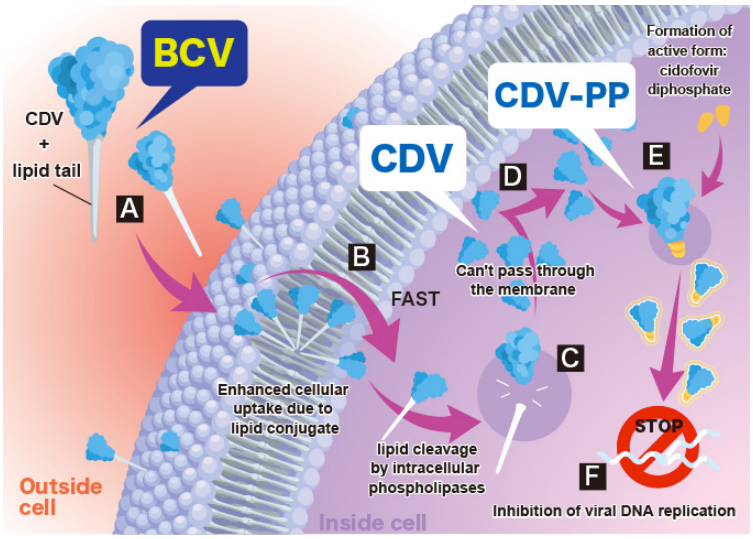
Source: Prepared by FISCO from the Company's results briefing material and website

(3) Brincidofovir (BCV) (Intravenous formulation/Oral formulation)

a) Overview of Brincidofovir and licensing agreement

Brincidofovir (BCV) is a lipid conjugate of cidofovir (CDV), which is known as a treatment of cytomegalovirus (CMV) retinitis. BCV is an antiviral drug candidate that has higher anti-viral activity and a superior safety profile in comparison with CDV. Lipid conjugatation allows for more efficient uptake of BCV into cells than CDV alone. Once inside target cells, the lipid chain is cleaved, releasing CDV, which is then converted to its active form of cidofovir diphosphate (CDV-PP), which fulfills the role of inhibiting viral DNA replication. For this reason, data showing that BCV has a much higher anti-viral replication effect than CDV and other anti-viral drugs have been obtained from in vivo tests and other studies. In terms of the safety profile, CDV has the side effect risk of strong nephrotoxicity, including the risk of renal dysfunction, through the accumulation of CDV in renal tubular epithelial cells. However, because the lipid conjugation of BCV brings no accumulation of CDV in renal tubular epithelial cells, BCV has the outstanding feature of reducing the risk of nephrotoxicity associated with CDV. CDV has been granted fast track designation by the U.S. FDA for the prevention of cytomegalovirus and the treatment of adenovirus and smallpox, while in Europe the EMA has granted orphan drug designation for the same viruses.

How BCV works



Source: Reprinted from the Company's website

Chimerix had been developing an oral formulation of BCV, but it had discontinued development because it did not obtain favorable results in Phase III clinical trials, including some side effects. Subsequently, Chimerix had been looking for a partner to whom it could license out BCV in order to concentrate its management resources in the anti-cancer agent field, while the Company was searching for new drug agents to license in. The timing was right for both companies, and they concluded a licensing agreement for the manufacture, marketing and development (excluding smallpox) of BCV. The main factors behind the Company's decision included the fact that BCV is used for rare diseases and underserved therapeutic areas, which matched the Company's targets for development, and because, like TREAKISYM®, the indicated diseases are in the hematologic disease field, so there would be no need for additional sales representatives, and thus there would be significant synergies in terms of marketing efficiency.

Chimerix has suspended development of an oral formulation, but the Company sees the reason for this as being that the absorption rate of the drug from the gastrointestinal tract was low. The Company believes that an intravenous formulation could have the same effect as an oral formulation at just 10% of the dosage, so it thinks there is a higher probability of success. This agreement covers both the intravenous formulation and the oral formulation, as the Company believes that going forward, there is a possibility that it can resolve the issues with the oral formulation through new improvements to the drug. Of the viral infectious diseases, the reason why smallpox alone is excluded from the agreement is that the U.S. government needs to maintain its ability to manufacture and stockpile a smallpox treatment independently within the country as a measure to counter bioterrorism.

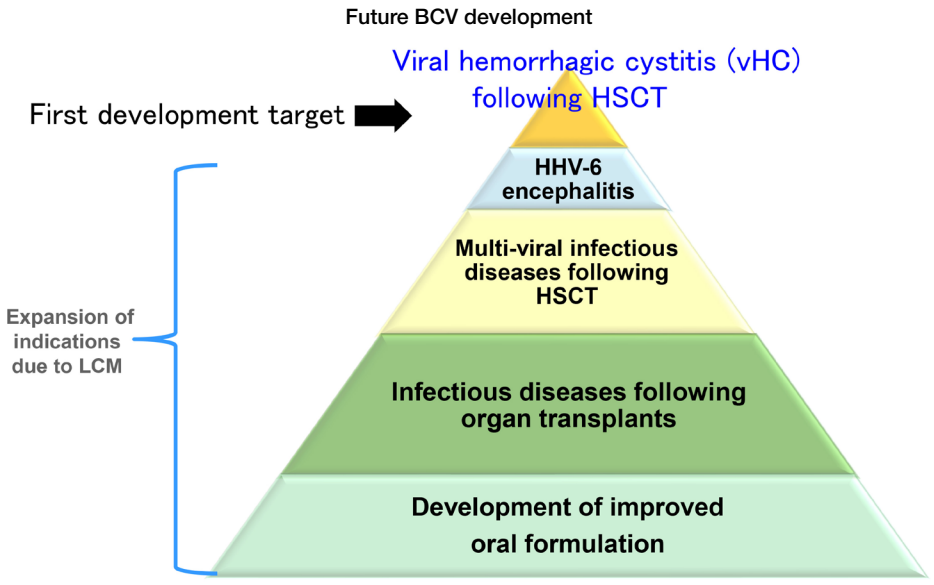
What is notable in this agreement is that it is a global license, and also that it covers manufacturing rights. This drug will be used to treat viral infectious diseases that occur following hematopoietic stem cell transplantation or organ transplants. The organ transplant market is large not only in the U.S. and Europe, but also in Asia, where there is significant growth potential. TREAKISYM® has been marketed in South Korea, Taiwan, and Singapore through sales partners. However, sales volumes were small and there was only a negligible impact on the Company's business performance. If the overseas rollout of BCV is successful, the Company will consider growth as a global company.

Also, the quality issue for TREAKISYM® that arose in FY12/19 impacted the decision to have the agreement include manufacturing rights. The Company understands the importance of controlling manufacturing on its own and building a system to suppress business risks to the maximum extent possible. The Company is currently in the process of selecting a manufacturing contractor, and this will be possible as long as the partner company has the technology to manufacture a highly-active anti-viral drug. It is expected that a decision will be made soon. In conjunction with the conclusion of the licensing agreement, the Company paid developer Chimerix an upfront payment of US$5mn (approximately ¥540mn) in Q3 FY12/19, and will pay future milestones of up to US$180mn (approximately ¥19.4bn), as well as a double-digit royalty on net sales of brincidofovir products.

b) Future development plans

As a future development plan, the Company is planning to first develop an intravenous formulation to treat viral hemorrhagic cystitis following hematopoietic stem cell transplantation in Japan. The Company is aiming to start a clinical trial in H2 FY12/20, and is targeting the launch of sales in 2025. Chimerix has already completed a Phase I clinical trial, so it appears the Company wants to start from a Phase II clinical trial citing the data from Chimerix's Phase I clinical trial. In addition, the Company is considering making use of the SAKIGAKE system, which can reduce the reviewing time needed for drug approval.

In Japan, there are roughly 3,700 hematopoietic stem cell transplants (allogeneic) in each year, and it is reported that the incidence rate for viral hemorrhagic cystitis (vHC) is between 8.6% and 24% (even higher for cord blood stem cell transplantation) for these transplants. Currently, there are no effective approved drugs, and in some cases, physicians are individually importing non-approved CDV, but these have side effects, so a safe treatment is desirable. By advancing clinical development in Japan before anywhere else in the world, the Company is looking to lay the foundation for a global business roll-out. The Company will decide its development plan regarding its overseas development strategy by Q3 FY12/20. On a global basis, there are approximately 35,000 hematopoietic stem cell transplants (allogeneic) performed each year, and when organ transplants are included, the number of procedures exceeds 100,000. Thus, the Company will be developing a treatment for a variety of viral infectious diseases that occur following these transplant operations. As for overseas business expansion, the Company plans to implement a partnership strategy that harnesses the regional characteristics of the targeted diseases.



Source: From the Company's results briefing material

In addition, the novel coronavirus pneumonia, which has continued to spread worldwide since February 2020, is an RNA virus infectious disease, and therefore basically falls outside the protective scope of BCV, which inhibits DNA viruses. However, despite its mechanism of action being unknown, it has been found effective as a treatment for Ebola hemorrhagic fever, also an RNA virus infectious disease. Therefore, the Company plans to contact Chimerix and have a reverification performed.

BCV's first development and commercialization target

Disease field	Indication	Current status	Problems
Hematopoietic stem cell transplants	Viral hemorrhagic cystitis (vHC)	• With no approved drug therapies in Japan, some physicians privately import cidofovir (CDV) and administer it to their patients. (The standard treatment method is to administer 5mg/kg weekly for two weeks, then subsequently administer the same dosage once every two weeks as maintenance therapy) • Bladder perfusion is the only treatment for symptoms. • Patients suffer from urinary problems and pain, and disseminated infections can be lethal.	• Bladder perfusion is the only treatment for symptoms.• No definitive treatments

Source: Prepared by FISCO based on the Company's materials

Estimated number of estimated allogeneic hematopoietic stem cell transplants and kidney plants

(Number of cases, people)

Region (population)	U.S. (320 million)	EU (550 million)	Japan (130 million)	Other	Total
Hematopoietic stem cell transplants (allogeneic)	8,700	16,400	3,700	6,454	35,254
Kidney transplants	19,860	20,000	1,648	39,052	80,560
Liver transplants	7,800	7,400	438	10,062	25,700
Other transplants	5,940	4,500	124	1,276	11,840

Source: Prepared by FISCO, based on the Company's materials prepared according to Chimerix's materials (January 2019)

Results trends

Decline in revenue in FY12/19 due to TREAKISYM® quality issue, increase in R&D expenses and costs to build the Company's own sales system

1. FY12/19 results

In FY12/19, net sales decreased 26.0% YoY to ¥2,837mn, the operating loss was ¥4,301mn (compared to a loss of ¥2,656mn in the previous year), the ordinary loss was ¥4,376mn (compared to a loss of ¥2,748mn in the previous year) and the loss was ¥4,376mn (compared to a loss of ¥2,752mn in the previous year). The main reason for the lower net sales was that the Company temporarily halted imports and sales of TREAKISYM® products that were imported from Astellas Deutschland GmbH, a subsidiary of Astellas Pharma Inc. <4503> because problems such as contaminants and appearance defects were found in these products. The Company procures TREAKISYM® products from suppliers with factories in Belgium and Germany. It looks like there were problems with the manufacturing process and quality control at the factory in Germany, which had been the Company's supplier. However, there were then also problems with products supplied by a factory in Belgium, and this resulted in net sales falling well short of the previous year's results as well as the Company's forecasts. With these quality issues, the Company is in ongoing talks with Astellas Deutschland GmbH, including demanding corrective measures, to have the company fulfill its obligation as a stable supplier, but it appears that the quality issue has not been completely resolved. It appears that the size of the end market for TREAKISYM® in 2019 on an NHI drug price basis was roughly flat year-on-year at approximately ¥8,400mn. While shipments from the Company declined, it appears that inventories held by sales partner Eisai and its pharmaceuticals wholesalers were sold.

On the cost front, R&D expenses rose 33.2% YoY to ¥2,441mn. The aforementioned upfront payment for BCV accounted for ¥540mn of these expenses, while the rest were related to clinical trials for TREAKISYM® and rigosertib. Other SG&A expenses increased 36.5% YoY to ¥2,724mn. This increase was mainly due to higher costs recorded to build the Company's own sales platform for TREAKISYM®. During Q4 FY12/19, the Company recruited additional TREAKISYM® regional sales managers and sales representatives, formed business alliances with pharmaceutical wholesalers, and moved to a system with two logistics centers (eastern Japan and western Japan), as well as bolstered information systems, with the aim of enhancing its distribution and logistics capabilities.

FY12/19 results

(¥mn)

	FY12/18 Results	FY12/19				
		Initial forecast	Results	YoY	YoY (%)	Vs. forecast
Net sales	3,835	4,465	2,837	-997	-26.0%	-1,627
Product sales	3,809	4,457	2,811	-998	-26.2%	-1,645
Other sales	25	7	26	0	3.2%	19
Gross profit	1,172	1,466	864	-308	-26.3%	-601
SG&A expenses	3,828	5,053	5,166	1,337	34.9%	113
R&D expenses	1,832	2,508	2,441	608	33.2%	-66
Other SG&A expenses	1,996	2,545	2,724	728	36.5%	179
Operating profit (loss)	-2,656	-3,587	-4,301	-1,645	-	-714
Ordinary profit (loss)	-2,748	-3,612	-4,376	-1,627	-	-764
Profit (loss)	-2,752	-3,616	-4,376	-1,623	-	-760

Source: Prepared by FISCO from the Company's financial results and results briefing material

Expecting net sales to recover and expenses to continue to increase in FY12/20

2. Outlook for FY12/20

The outlook for the FY12/20 results is for net sales to increase 20.0% YoY to ¥3,404mn, an operating loss of ¥5,090mn (compared to a loss of ¥4,301mn in FY12/18), an ordinary loss of ¥5,134mn (a loss of ¥4,376mn), and a loss of ¥4,803mn (a loss of ¥4,376mn). Net sales are expected to increase YoY for the first time in two years due to the recovery in the volume of TREAKISYM® procured.

Meanwhile, on the cost front, R&D and other SG&A expenses are expected to increase continuously. With respect to R&D expenses, the initial payment of ¥540mn in conjunction with the BCV agreement will disappear, but the Company is expecting to pay a milestone payment of ¥500mn based on the marketing approval of the RTD formulation of TREAKISYM® (liquid formulation), as well as the addition of clinical trial expenses for BCV, on top of those for TREAKISYM® and rigosertib, so the Company is forecasting a 11.9% YoY increase to ¥2,731mn.

Also, other SG&A expenses are expected to increase 28.6% YoY to ¥3,505mn, mainly due to expenses to establish the Company's own sales platform. The Company plans to complete the building of its sales system, logistics system, and internal information system by Q2 FY12/20, and expects to commence its own sales immediately following the expiration of the marketing agreement in December 2020. In terms of the salesforce, the Company will divide the nation into six blocks, and build a locally-rooted platform with 62 people (including approximately 30 regular employees and 30 contract MRs). From April 2020 onward, the Company plans to hire 30 contract MRs and provide them with training. Concerning internal information systems, including a system to manage orders, the Company is currently conducting test operations with the goal of operation from H2, and is otherwise preparing to be able to quickly transition to the new system after the agreement with Eisai expires in December 2020.

Outlook for FY12/20

(¥mn)

	FY12/19 Results	FY12/20 Company forecast	Change	% change
Net sales	2,837	3,404	566	20.0%
Gross profit	864	1,146	281	32.5%
SG&A expenses	5,166	6,236	1,069	20.7%
R&D expenses	2,441	2,731	289	11.9%
Other SG&A expenses	2,724	3,505	780	28.6%
Operating profit (loss)	-4,301	-5,090	-788	-
Ordinary profit (loss)	-4,376	-5,134	-757	-
Profit (loss)	-4,376	-4,803	-426	-

Source: Prepared by FISCO from the Company's financial results and results briefing material

In October 2017, the Company filed a petition for arbitration with the International Chamber of Commerce (ICC) in the U.S. against The Medicines Company for damages for the non-fulfillment of a licensing agreement.* The final documents were submitted in December 2019, and the outcome will likely be announced within three to six months if the case proceeds as usual. The claim was for US$82mn, and the decision announced by the ICC will effectively be the final confirmation, but the impact of this has not been included in the Company's forecasted results. Novartis AG (Switzerland) announced in January 2020 that had made The Medicines Company a subsidiary, so there is no risk that compensation for damages will become unpayable.

* In 2015, the Company concluded a licensing-in agreement with The Medicines Company for a self-administered, short-term acute pain management medication. The Company, acting in the best interests of patients, temporarily suspended new patient enrollment for the drug from April 2017 due to the concern about the continuity of The Medicines Company's business regarding the product. In October 2017, the Company filed for arbitration with the ICC seeking a payment of US$82mn as compensation for damages for the non-fulfillment of the licensing agreement. In the arbitration, the Company claims that The Medicines Company was not able to provide the Company with adequate assurance of its performance of obligations under the licensing agreement in light of its decision to discontinue commercialization activities regarding the product and withdraw from markets in the U.S. and Europe, and that such failure by The Medicines Company was a material breach of the licensing agreement. The Company then terminated the licensing agreement.

Issued stock acquisition rights to procure funds for business activities, the required procurement amount could change depending on the outcome of the ICC arbitration

3. Financial condition

Looking at the financial condition at the end of FY12/19, total assets stood at ¥5,273mn, a decrease of ¥965mn from the end of the previous fiscal year. The main factors behind this change were a ¥137mn increase in accounts receivable-trade, a ¥910mn decrease in cash and deposits, and a ¥533mn decline in merchandise and finished goods under current assets. For merchandise and finished products, assets were previously booked at the time of shipment from Astellas Deutschland's factories, but due to the quality issue that arose, this process was changed so that assets are booked after completion of an examination upon arrival. Under non-current assets, there was a combined increase of ¥169mn in software and software in progress related to the Company's effort to build its own sales system.

Total liabilities stood at ¥873mn, an increase of ¥463mn from the end of the previous fiscal year. The main factor behind this change was an increase of ¥136mn in accounts payable-other, which was partly offset by a decrease of ¥605mn in accounts payable-trade. In addition, total net assets stood at ¥4,400mn, a decrease of ¥501mn from the end of the previous fiscal year. While capital stock and capital surplus totaled ¥3,798mn following the exercise of stock acquisition rights, retained earnings decreased ¥4,376mn due to the recording of a loss. Consequently, the equity ratio decreased from 70.1% at the end of the previous fiscal year to 71.7%.

The Company issued the 50th and 51st Stock Acquisition Rights (with Exercise Price Revision Clauses) via an allotment to EVO FUND on March 16, in order to raise funds for business activities through H1 FY12/21. These rights represent 10 million dilutive shares, and if all of the rights are exercised, shareholder value per share would be diluted by 37.8%. However, if the Company receives compensation for damages based on the aforementioned arbitration with The Medicines Company, the Company can limit the number of rights exercised depending on the amount received from the arbitration. The amount of funds to be raised is approximately ¥5.4bn, and if more than a certain amount of compensation for damages are received, the Company can at that time buy back and cancel all non-exercised stock acquisition rights. Therefore, the outcome of the arbitration is of high interest.

Balance sheet and management indicators

(¥mn)

	End-FY12/16	End-FY12/17	End-FY12/18	End-FY12/19	Change
Current assets	6,685	4,036	6,038	4,887	-1,150
(Cash and deposits)	5,719	2,947	4,821	3,910	-910
Non-current assets	193	215	200	386	185
Total assets	6,878	4,252	6,239	5,273	-965
Total liabilities	1,393	1,012	1,337	873	-463
(Interest-bearing debt)	450	-	-	-	-
Net assets	5,484	3,239	4,901	4,400	-501
Management indicator					
Equity ratio	73.5%	63.6%	70.1%	71.7%	

Source: Prepared by FISCO from the Company's financial results

Overview on the issuance of acquisition rights by third-party allotment

	50th Stock Acquisition Rights	51st Stock Acquisition Rights
Total number of stock acquisition rights	700	300
Number of dilutive shares from the issuance	700	300
Exercise price and conditions for revising the exercise price	94% of the simple average value of the volume weighted average price on each trading day for the five consecutive trading days prior to the Revision Date. Minimum exercise price is ¥291	
Expected exercise period	In principle, commitment to the exercise all Stock Acquisition Rights within five months following issuance (commitment to the exercise of at least 40% of issued Stock Acquisition Rights within 56 trading days) Excluding the case in which an event triggering the extension of the commitment period occurs	In principle, commitment to the exercise of all Stock Acquisition Rights within 46 trading days from the exercise start date specified by the Company Excluding the case in which an event triggering an extension of the commitment period occurs
Expected start date of exercise	March 17,2020	To be determined
Expected completion date of full commitment	August 21,2020	To be determined
Amount to be funded (¥mn)	3,829	1,641

Source: Prepared by FISCO from the Company's news release

Uses of the funds

(¥mn)

	50th Stock Acquisition Rights	51st Stock Acquisition Rights	Expected timing of expenditure
Development of in-licensed drugs	2,375	55	March 2020 to June 2021
Creation of the Company's own sales platform	1,431	54	March 2020 to June 2021
Investment in new in-licensing, M&A, and other	0	1,535	October 2020 to June 2021
Total	3,806	1,644	

Source: Prepared by FISCO from the Company's news release

Mid-Range Plan

Targeting a turn to profitability in FY12/21 and to continuously have an operating profit margin of at least 10%

1. Mid-Range Plan

In February 2020, the Company announced a Mid-Range Plan that spans the three years through FY12/22. In the Plan, the Company aims to have net sales of ¥10,816mn and operating profit of ¥1,482mn in FY12/22, and to secure an operating profit margin of at least 10% on a continual basis. In FY 12/21, the Company aims to begin full operation of its own sales platform. Based on this, by quickly switching from the current lyophilized powder formulation type of TREAKISYM® to the RTD formulation of TREAKISYM®, and by expanding market penetration with the additional indication of to treat relapsed and refractory DLBCL to quickly achieve annual net sales of ¥10bn on an NHI price basis, the Company hopes to achieve an operating profit in FY12/21 and thereafter realize sustainable growth. With the addition of BCV to the development pipeline, the Company aims to achieve growth as a global specialty pharmaceutical company.

Mid-Range Plan

(¥mn)

	FY12/19 results	FY12/20 forecast	FY12/21 targets	FY12/22 targets
Net sales	2,837	3,404	9,008	10,816
Operating profit (loss)	-4,301	-5,090	1,031	1,482
Ordinary profit (loss)	-4,376	-5,134	987	1,438
Profit (loss)	-4,376	-4,803	1,356	1,717

Source: Prepared by FISCO from the Company's results briefing material

2. Assumptions behind Performance Targets

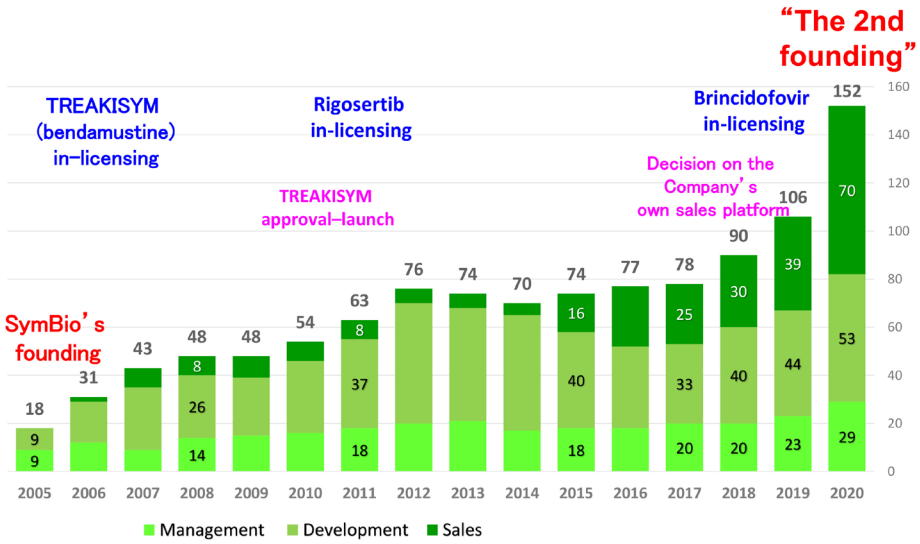
The majority of net sales are product sales for TREAKISYM®. The sales targets take into account recent sales trends and the pace of market penetration. Net sales through FY12/20 are sales based on product shipment unit prices to Eisai, but from FY12/21, sales will be based on shipment unit prices to pharmaceutical wholesalers by the Company's own sales platform, so net sales are expected to rapidly increase. The main factor for the increase in sales in FY12/22 is the increase in demand because of the additional indication of relapsed and refractory DLBCL, for which approval is expected in FY12/21.

The Company is expecting gross profit margin to increase from the FY12/20 forecast of 33.7% to 70–80% in FY12/22 due to the shift to its own sales platform and progress in the switch to the RTD formulation.

The Company is forecasting SG&A expenses of ¥6,236mn in FY12/20, and forecasts SG&A expenses to remain around the ¥6.0bn level from FY12/21 onward (excluding milestone payments). Of this, R&D expenses are estimated based on the Company's plans for development plan for its existing pipeline, comprising TREAKISYM®, the rigosertib intravenous and oral formulations, and BCV, but expenses such as initial payments on agreements related to the new pipeline are not included. Other SG&A expenses mainly consist of expenses for sales and marketing, production and logistics operations, business development, and management related to TREAKISYM®, and the Company expects the main cost increases to be for personnel and associated expenses resulting from the increase in medical representatives. The Company is planning to increase its workforce from 106 people at the end of 2019 to 152 people at the end of 2020, but is not planning any more significant increases in personnel thereafter.

Mid-Range Plan

Number of employees



Source: From the Company's results briefing material

From FY12/21 onward, profit exceeds ordinary profit. This is due to the fact that the Company reflected the impact amount of the reduction of losses carried forward in conjunction with becoming profitable in tax-effect accounting.

Sales growth potential will be even larger if the global roll-out of BCV proceeds well

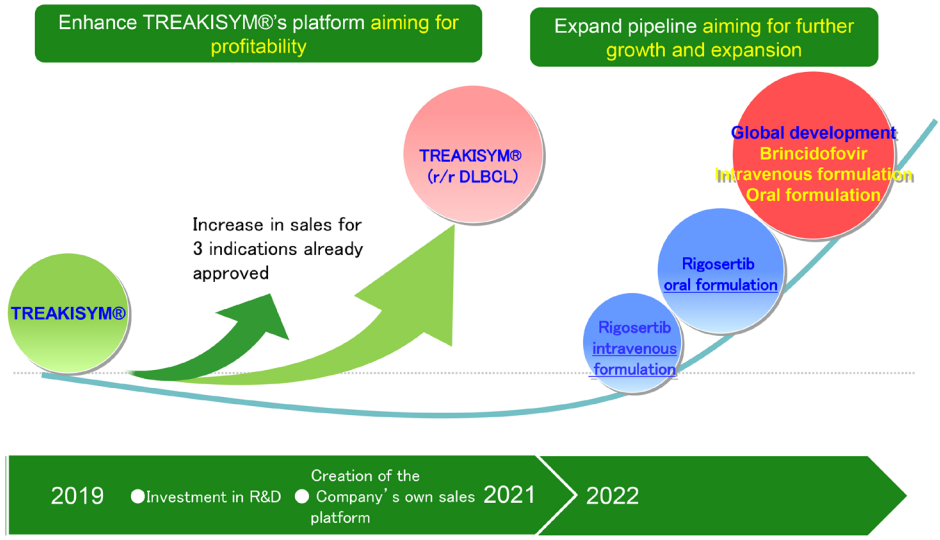
3. Sales growth potential

Looking at sales growth potential, if marketing approval of TREAKISYM® for the indication of relapsed and refractory DLBCL is obtained, the number of potential patients in Japan will roughly double all at once. Estimates of potential sales will vary depending on what percentage is set for the market penetration rate. Excluding DLBCL, potential sales are estimated at around ¥12bn to ¥13bn on a drug-price basis. If simply calculated, potential sales are expected to approximately double to ¥24bn to ¥26bn by adding patients with relapsed and refractory DLBCL.

Moreover, if rigosertib is approved for an indication for untreated high-risk MDS, it can be expected to achieve sales of around the same scale of azacitidine (approximately ¥15bn). The sales growth potential for both drugs will increase from ¥8.5bn in 2018 to around ¥40bn on an NHI drug price basis, while the Company's net sales will amount to more than ¥30bn, but the addition of BCV as a new pipeline drug has increased the growth potential even more. As discussed above, BCV stands out for its strong anti-viral action against multiple DNA viruses, and there are strong hopes for its development as a drug to treat viral infectious diseases following hematopoietic stem cell transplantation and organ transplants. If its global roll-out is successful, the sales growth potential could exceed ¥100bn, which makes it a drug of high interest going forward.

Mid-Range Plan

Illustration of “The 2nd SymBio”



Source: From the Company's results briefing material

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■ For inquiry, please contact: ■
FISCO Ltd.
5-11-9 Minami Aoyama, Minato-ku, Tokyo, Japan 107-0062
Phone: 03-5774-2443 (Financial information Dept.)
Email: support@fisco.co.jp