COMPANY RESEARCH AND ANALYSIS REPORT

SymBio Pharmaceuticals Limited

4582

JASDAQ Growth Market

30-Oct.-2020

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Summary

Completed building its own TREAKISYM® sales platform toward becoming profitable in FY12/21

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 Company concluded a global licensing agreement with Chimerix <CMRX> for the antiviral drug Brincidofovir (BCV), and its policy is to newly develop its business overseas as well, with the aim of becoming a global specialty pharmaceutical company in the future.

1. Strategies to become 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®. Up to the present time, it has made steady progress for all three strategies. For TREAKISYM®, the Company will shift to its own sales platform after the end of a sales agreement with Eisai Co., Ltd.<4523> in December 2020, and by Q2 FY12/20, it had completed recruiting highly specialized sales and marketing human resources, introducing the mission-critical system, constructing its own logistics system, and establishing a call center, so the preparations have been put in place. Also, it announced that it had received marketing approval for the RTD formulation on September 23, 2020, and it plans to start sales in January 2021. By making progress in switching from the existing lyophilized powder formulation type, it is aiming to quickly achieve net sales of ¥10bn on a drug-price basis. Though launching its own sales and switching to the RTD formulation, the TREAKISYM® profit margin is expected to improve. In addition, if its indication is expanded to relapsed and refractory diffuse large B-cell lymphoma (DLBCL), for which it is currently applying for marketing approval, the number of patients for which it is indicated will approximately double compared to previously. The outlook for the time period for acquiring approval is from Q3 FY12/21, and if steady progress is made, the Company is expected to achieve an operating profit in FY12/21.

2. Trends in the other development pipeline

For rigosertib (intravenous formulation), on August 24 the U.S. licensor Onconova Therapeutics, Inc.<ONTX> (hereafter, Onconova) announced the top-line (primary endpoints) data for the global joint phase III clinical trial for high risk MDS. Data clarified that a significant difference with the comparison group was not obtained for overall survival, which is the primary endpoint, and going forward it plans to conduct a detailed statistical analysis. Also, for BCV (intravenous formulation) licensed from Chimerix, based on the opinion of the global advisory board, the Company has firmed its policy for a global joint phase II clinical trial indicated for adenovirus infection (for children) that develops after hematopoietic stem cell transplantation and for which there is still no treatment, and it has started preparations. If approval is acquired for the adenovirus infection indication, it will expand the indications to other DNA viral infections and progress its development as a treatment and preventative drug for multi-viral infections after hematopoietic stem cell transplantation and infections after organ transplantation. The Company has acquired manufacturing rights for BCV and is currently in the process of selecting a manufacturing contractor ahead of clinical trials.

3. Results trends

In H1 FY12/20 (January to June 2020) results, net sales decreased 32.1% year-on-year (YoY) to ¥1,360mn and operating loss was ¥1,839mn (compared to a loss of ¥2,015mn in the same period in the previous fiscal year). Net sales decreased mainly due to the prolonging of a quality issue at the factory of the TREAKISYM® supplier, and the amount supplied decreased. Expenses increased to strengthen the inspection system in Japan, but the operating loss still shrank as the Company worked to keep down R&D expenses and other SG&A expenses.

The Company revised the FY12/20 results forecasts on September 17, 2020. While the net sales forecast was reduced 10.6% compared to the previous forecast to ¥3,043mn, the extent of the operating loss was reduced to ¥4,592mn from the previous forecast of ¥5,090mn. This is because, excluding the necessary investment toward becoming profitable in FY12/21 and BCV development expenses, the Company reviewed all other expenses and greatly reduced SG&A expenses from the initial forecast. Also, it filed a petition for arbitration with the International Chamber of Commerce (ICC) in the U.S. against The Medicines Company (U.S.) for the non-fulfillment of a licensing agreement. The ICC announced its final decision for the arbitration on September 1, 2020, and the Company will receive from the Medicines Company 50% of its expenses relating to the arbitration proceedings, including attorneys' fees (US$4.95mn). Based on this, it reduced the net loss forecast to ¥3,796mn from the previous forecast of ¥4,803mn.

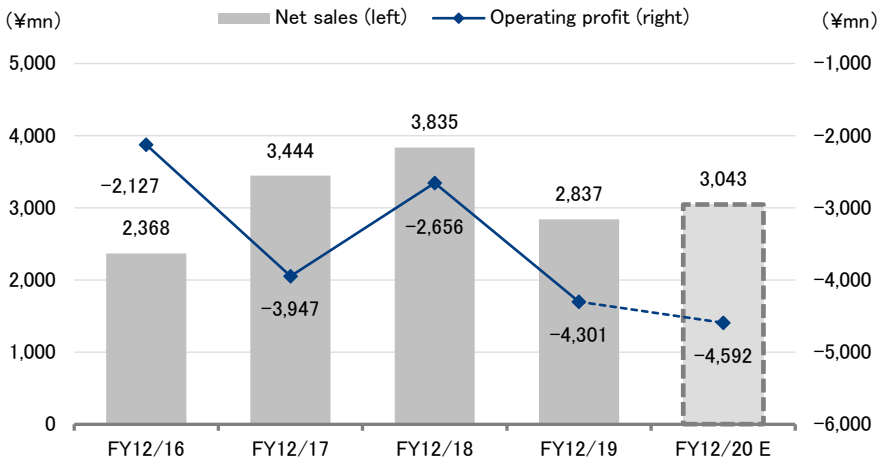
4. Mid-Range Plan

The Company's targeted results in the Mid-Range Plan are net sales of ¥10,816mn and operating profit of ¥1,482mn in FY12/22, and it is thought to be aiming for an operating profit margin that is continuously at least 10%. However, on considering the impact of the revisions to the FY12/20 full year results forecast, it is closely considering the values in the Mid-Range Plan and intends to again disclose revised values. It plans to announce a new Mid-Range Plan in February 2021, and within it, it is believed that it will clarify the development strategy in the future not only for TREAKISYM®, but also for rigosertib and BCV.

Key Points

- In the H1 FY12/20 results, sales declined by double digits due to the continuing TREAKISYM® quality issue, but completed the construction of the sales and logistics systems toward becoming profitable
- The loss amount in FY12/20 may decrease from the initial forecast
- 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

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 is 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 it is 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.

* Following Eisai's change of business strategy, the licensing agreement will be terminated on December 9, 2020, after which time the Company will shift to its own sales platform domestically.

TREAKISYM®, whose sales were launched in Japan in December 2010, continued to be subsequently developed 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. 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 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. <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 has the advantage of greatly reducing 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 licensing agreement with Chimerix for BCV, which gives it the rights to develop, manufacture, and commercialize BCV for all viral diseases excluding smallpox. The features of BCV are that compared to cidofovir (CDV: unapproved in Japan), it has high antiviral efficacy and safety, as well as being effective against multiple DNA viruses. The Company has decided on a policy of first starting development from the global joint phase II clinical trial indicated for adenovirus infection after 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

Date	Summary
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)
September 2020	In the final arbitration ruling for the claim for damages filed against the Medicines Company (U.S) due to the non-fulfillment of a licensing agreement, the Company will receive from the Medicines Company 50% of its expenses relating to the arbitration proceedings, including attorneys' fees.

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

Trends in the development pipeline

The number of patients for which TREAKISYM® is indicated will approximately double by expanding its indications to relapsed and refractory DLBCL

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

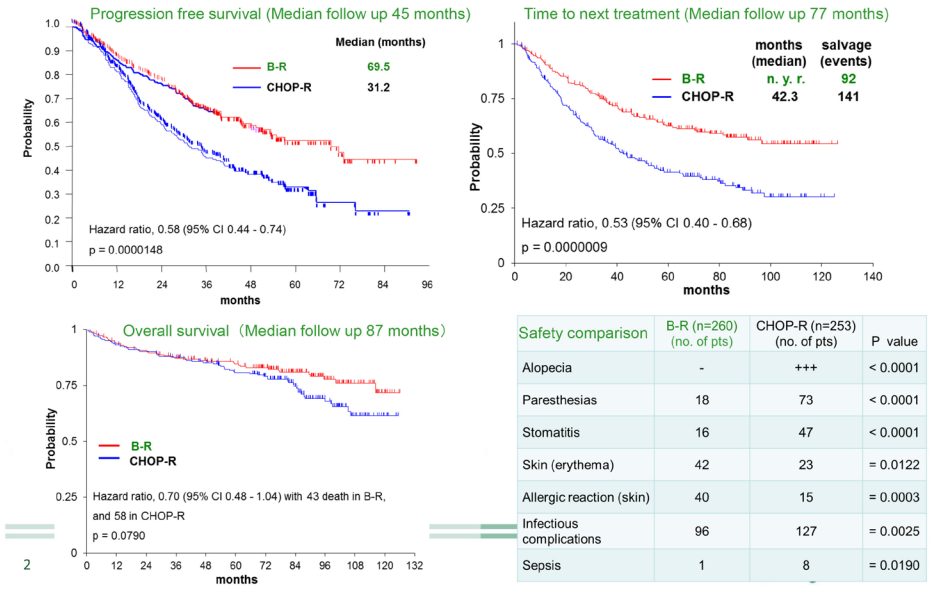
Type according to grade	Non-Hodgkin's lymphoma type (disease type)
Low grade: Indolent lymphoma (progresses yearly)	Follicular lymphoma (grade 1, 2), MALT lymphoma, lymphoplasmacytic lymphoma Mycosis fungoides, Sezary syndrome, chronic lymphocytic leukemia / small lymphocytic lymphoma, etc.
Medium grade: 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 Japan 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 has come to be positioned 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 the untreated area at the end of 2020. Also, it is currently progressing the following three therapies in the development pipeline.

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

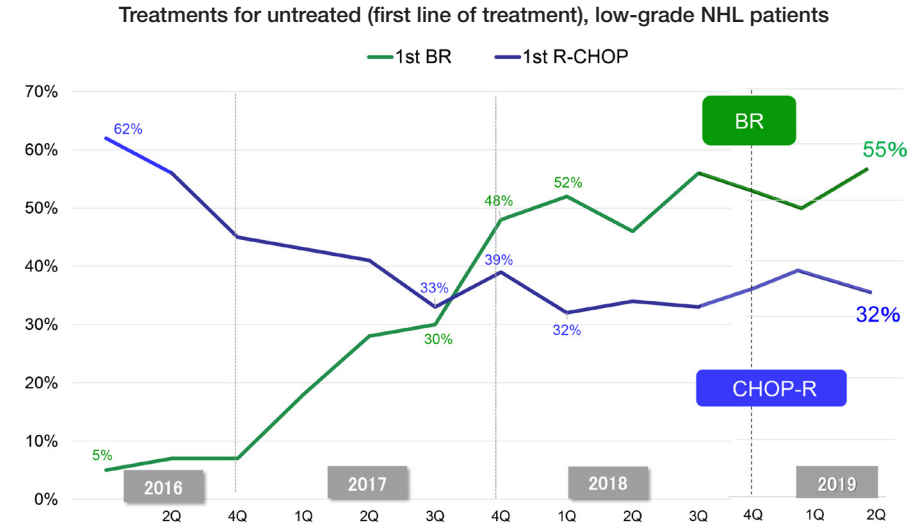
BR therapy It is superior to R-CHOP for both efficacy and safety



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

Trends in the development pipeline



Source: From the Company's results briefing material

(1) Expanding indications to relapsed and refractory DLBCL

Toward expanding the indications of the lyophilized powder formulation type TREAKISYM®, in the phase III clinical trial for BR therapy indicated for relapsed and refractory DLBCL, the observation periods for all of the subjects were completed in September 2019, and for the response rate, which is a primary endpoint, the result*1 exceeded the expected response rate. Based on this, the Company applied for approval in May 2020 for partial changes relating to the manufacturing and marketing approval items. Also, in June of the same year, Chugai Pharmaceutical Co., Ltd. <4519> applied for manufacturing and marketing approval for a combination therapy of Polatuzumab vedotin*2 and BR therapy indicated for relapsed and refractory DLBCL. Based on this, in July of the same year the Company also applied to change the approval to a combination therapy.

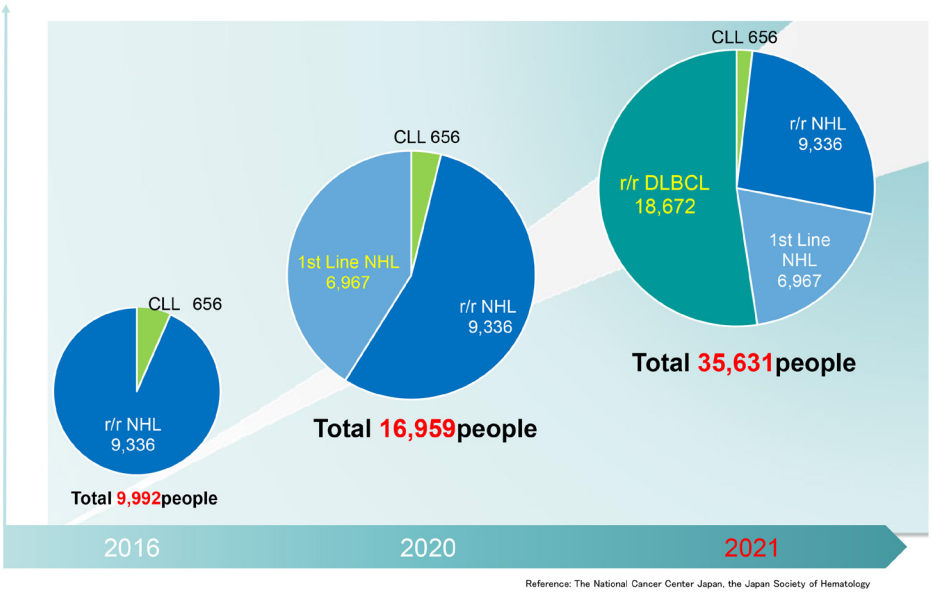
*1 For the total number of cases (38 cases), the complete response rate (CR) was 47.4% and the partial response rate (PR) was 28.9%. By age, CR was 71.4% and PR was 14.3% for subjects aged under 65 years, CR was 45.0% and PR 30.0% for 65 to 75 years, and CR was 36.4% and PR 36.4% for 76 years and above. In particular, achieving a CR level of 36.4% for subjects aged 76 years and above surprised the medical specialists.

*2 A first-in-class anti-CD79b antibody drug conjugate developed by Roche using Seagen's ADC technology. The CD79b protein is expressed specifically in many B cells, and it is considered that Polatuzumab vedotin binds to CD79b while suppressing the effects on the normal cells and that the B cells are destroyed by the delivered chemical therapy.

There is currently no effective treatment for relapsed and refractory DLBCL, while in palliative chemotherapy, there are strong side effects from a multi-drug therapy that combines several anticancer agents (3 to 6 types). Therefore, there has been a need for new, highly effective therapeutic agents and treatments with few side effects. Currently, if the BR therapy and the Polatuzumab vedotin+BR therapy are approved, this need may be met. If steady progress is made, it is anticipated that sales can start from Q3 FY12/21. In Japan, there are approximately 18,000 patients with relapsed and refractory DLBCL, which is more than the around 17,000 patients for the indications for which TREAKISYM® has already been approved, so the potential market scale will approximately double from previously. Patient advocacy groups and relevant academic societies have also filed petitions urging 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.

Trends in the development pipeline

Market expansion from the r/r DLBCL additional indication



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

(2) RTD formulation and RI formulation

On September 23, 2020, the Company announced that it had acquired manufacturing and marketing approval for the RTD formulation, which is the liquid formulation-type TREAKISYM®, and it intends to launch sales from January 2021. It 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. In the United States, the RTD formulation achieved a market penetration rate of 97% in one year from its sales launch*, so this is considered to be a feasible pace.

* In the United States, sales of the RTD formulation BENDEKA® were launched from January 2016, and it had achieved a market penetration rate of 70% by May of the same year and more than 90% after one year.

Also, for the RI formulation, it was announced on September 9, 2020, that in the clinical trial started in November 2018 (scheduled number of cases, 36 cases), the observation periods of all the subjects had been completed. If steady progress is made, approval is expected to be acquired in H2 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.

As previously stated, an advantage of the RTD/RI formulation is that it greatly reduces the workload on medical practitioners by eliminating the need to dissolve the drug, while the RI formulation also greatly reduces the burden on the patient by shortening the administration time to 10 minutes (compared to 60 minutes for the existing drug and the RTD formulation). The exclusive sales period for the existing lyophilized powder formulation type ends in Japan in 2020, so generics may be developed for it. But if the RTD/RI formulation is market launched, the difference in terms of functions will be large, so in actual terms, it will be possible to extend the exclusive sales period until 2031. In the event that the RTD/RI formulation is market launched, the drug price will be the same level as the previous drug. But at FISCO, we think that because the supplier will change to Eagle Pharmaceuticals, it is highly likely that the profit margin will improve compared to that of the existing drug. Also, due to the quality defect issue that has occurred since 2019 for the lyophilized powder formulation, from the viewpoint of business continuity risk the Company has in sight for the future the in-house manufacturing of the RTD/RI formulation. This seems possible in terms of the agreement, and it is thought it will outsource manufacturing 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	Applied for marketing approval in May 2020
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. Patient enrollment was completed in March 2020 and plans to apply for approval in the second half of 2022.

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

From the clinical results of rigosertib indicated for relapsed and refractory high risk MDS, no significant difference was obtained for the primary endpoint

2. Rigosertib (Intravenous formulation/Oral formulation)

Rigosertib is a therapeutic drug candidate 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 mainly being progressed by its licensor, Onconova, indicated for high-risk myelodysplastic syndrome (below, MDS).

In this development, on August 24, Onconova announced the topline data for the global joint phase III clinical trial indicated for relapsed and refractory high-risk MDS (intravenous formulation), which is a development project that the Company is also participating in. For overall survival, which is the primary endpoint of this clinical trial, it was 6.4 months for rigosertib + the best support therapy, and 6.3 months (p=0.33) for the doctor selected therapy + the best support therapy, so it was clarified that there was no significant difference. Also, no significant difference between the two groups was observed for safety. Based on these results, going forward Onconova plans to conduct a detailed analysis and to utilize the findings obtained from a genome analysis for new developments.

For the development of the oral formulation, following the changes to Onconova's development strategy, the project indicated for high-risk MDS has declined in the order of priorities. But based on the results of the current intravenous formulation trial, discussions are being progressed with Onconova about the development policy in the future. Onconova announced that from June 2020, it will start a doctor-led phase 1/2a clinical trial indicated for progressive KRAS* positive NSCLC (non-small cell lung cancer) as a new indication for the oral formulation. This is because the results of the pre-clinical trial suggested it was effective in combination with an immune checkpoint inhibitor.

* 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 single drug	P3 global trial has been completed and the topline data was announced by Onconova in August 2020. There was no significant difference for the primary endpoint and going forward, it plans to conduct a detailed analysis.
SyB L-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

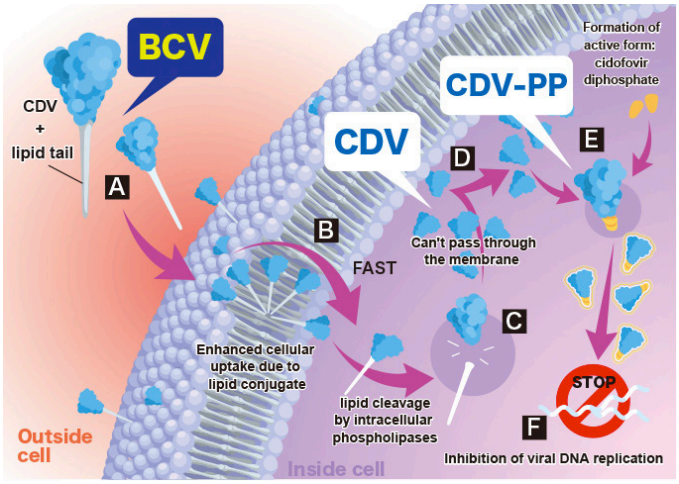
Decided on a policy for Brincidofovir of starting from a global clinical trial indicated for adenovirus infection after hematopoietic stem cell transplantation

3. Brincidofovir (Intravenous formulation/Oral formulation)

(1) 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 whose features are that it has higher anti-viral activity, exhibits anti-viral activity against a wider range of viruses, and has a superior safety profile compared to CDV. BCV has a structure that conjugates a lipid chain into CDC, which 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 discontinued development because it did not obtain statistically significant results in the phase III clinical trials, and also as there were some side effects, including diarrhea. Subsequently, Chimerix was looking for a partner to whom it could license out BCV 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 in September 2019 they concluded a licensing agreement for the global 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. Chimerix plans to apply to the FDA for marketing approval during 2020.

What is notable in this agreement is that it is a global license, and it also 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, and the potential market scale is significant as well. 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, it will enable it to grow as a global specialty pharmaceutical 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, and it will make a decision up to the clinical trial. 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 it 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.

(2) Future development plans

For the BCV (intravenous formulation) development strategy, the Company decided on a policy for the future in the global advisory board meeting held in February 2020. Specifically, it decided on the following three strategies: it will progress development that utilizes its multi-viral activity, which is a strength of BCV; it will target multiple infections, including the adenovirus for which there is no treatment and for which medical needs are high; and as the top priority, it will conduct development in the field of transplants for children, for which medical needs are high.

Trends in the development pipeline

As the first development target, the Company decided to start from the global joint phase II clinical trial (Japan, the U.S., and Europe) indicated for adenovirus infection after hematopoietic stem cell transplantation, acceding to the strong wishes of the board's medical specialist members. Safety can be skipped as the data from the clinical trials conducted by Chimerix (on more than 1,000 subjects) can be utilized. The adenovirus is a virus that is present in nature, and it causes infectious diseases such as pharyngitis, tonsillitis, conjunctivitis, gastroenteritis, and hemorrhagic cystitis through respiratory, eye, intestine, urinary, and other infections. If a healthy person is infected, serious cases are rare, but there is a high risk that symptoms will become severe in patients who have undergone a hematopoietic stem cell transplantation and whose immune systems have been weakened, and as yet there is no treatment for it. Therefore, the situation is that there is a strong need to develop treatments and preventative drugs. Although there are few cases, the Company is first progressing development indicated for children and after that, it plans to expand the indication to adults as well. It intends to decide on the specific development schedule and other matters in the future. The Company is targeting 2026 in Japan and around 2027 overseas as the approximate sales launch periods.

Globally, the number of hematopoietic stem cell transplantations (allogeneic transplantations) is 35,000 cases a year, of which, around 3,700 cases are in Japan. Looking by age in Japan, the number of transplantations for patients aged 19 years and younger is around 500 cases, which constitutes about 14% of the total*1. On estimating the same rate for overseas, 4,000 to 5,000 cases will be for patients aged 19 years and younger. There is no clear data on the number of cases of adenovirus infection after hematopoietic stem cell transplantation, but in past literature in Japan, it has been reported that the incidence of hemorrhagic cystitis caused by adenovirus infection is around 3.5%*2.

*1 Source: The Japanese Data Center for Hematopoietic Cell Transplantation, "Activities and Outcomes of Hematopoietic Cell Transplantation in Japan (2019)"
*2 Source: Clinical and Experimental Nephrology 2008: 50 (8) Analysis of acute necrotizing tubulointerstitial nephritis due to adenovirus infection after hematopoietic stem cell transplantation

Estimated numbers of (allogeneic) hematopoietic stem cell transplantations and organ transplantations

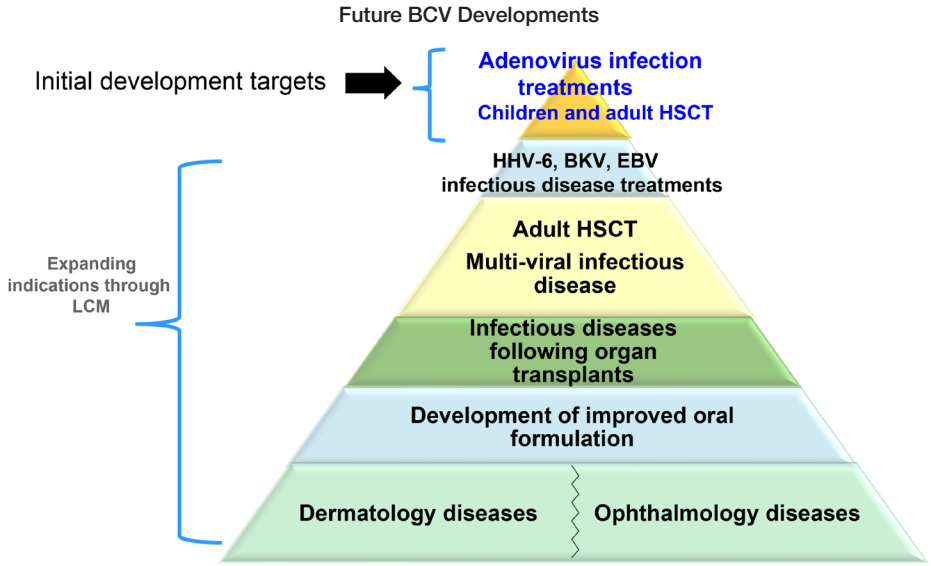
(Number of cases, people)

Region (population)	U.S. (320 million)	EU (550 million)	Japan (130 million)	Other	Total
Hematopoietic stem cell transplantation (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)

If the Company succeeds with its development for the adenovirus infection, it is expected to widen the fields to other viral infections as well, to establish a position for treatments and preventative drugs for multi-viral infections after hematopoietic stem cell transplantation, and then after that, to improve the development for infections after organ transplantations and for an oral formulation. Also, as viral infections are present in the dermatology and ophthalmology fields, in the future it is possible it will expand the scope to carry out joint development with pharmaceutical companies conducting development in these specialist fields.

Trends in the development pipeline



Source: From the Company's results briefing material

Results trends

In the H1 FY12/20 results, the TREAKISYM® quality issue continued and sales declined by double digits but completed the construction of the sales and logistics systems toward becoming profitable

1. Overview of the H1 FY12/20 results

In the H1 FY12/20 results, net sales declined 32.1% YoY to ¥1,360mn, the operating loss was ¥1,839mn (compared to a loss of ¥2,015mn in the same period in the previous fiscal year), the ordinary loss was ¥1,883mn (a loss of ¥2,069mn), and the loss was ¥1,884mn (a loss of ¥2,069mn).

H1 FY12/20 results

(¥mn)

	H1 FY12/19	H1 FY12/20	Change	% change
Net sales	2,004	1,360	-644	-32.1%
Gross profit	529	329	-199	-37.7%
SG&A expenses	2,544	2,169	-374	-14.7%
R&D expenses	962	833	-128	-13.4%
Other SG&A expenses	1,581	1,336	-245	-15.5%
Operating profit (loss)	-2,015	-1,839	175	-
Ordinary profit (loss)	-2,069	-1,883	186	-
Extraordinary profit/loss	1	-	-1	-
Profit (loss)	-2,069	-1,884	184	-

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

Net sales decreased mainly because, continuing on from the previous period, there was no improvement to the defective quality issue (contamination and poor external appearance) in products imported from the European subsidiary (Astellas Germany) of Astellas Pharma Inc. <4503>, and the amount shipped was limited. The Company has strongly demanded from Astellas that it re-investigate the root cause of and improve this quality issue. Also, to prevent a product recall due to poor quality, it has strengthened the post-import inspection system (doubled the visual inspections), which became a factor causing the cost ratio to worsen. The Company estimates that the impact of the quality issue has been to reduce gross profit by approximately ¥270mn. For those products it determined could not be sold due to their poor quality, it recorded ¥68mn as an inventory write-down (recorded ¥187mn in the same period in the previous fiscal year).

SG&A expenses decreased 14.7% YoY to ¥2,169mn. Within this amount, R&D expenses declined 13.4% to ¥833mn due to the reduction in the clinical trial expenses of TREAKISYM® and rigosertib. Other SG&A expenses decreased 15.5% to ¥1,336mn due to the measures to reduce expenses, including a hiring freeze, and as a result, the operating loss contracted ¥175mn compared to the same period in the previous fiscal year.

Net sales of TREAKISYM® by Eisai during the same period increased slightly YoY, from ¥3.4bn to ¥3.5bn, and it appears that final demand for TREAKISYM® is trending steadily. The Company's sales agreement with Eisai will end in December 2020, after which it will switch to its own sales platform. It progressed the construction of its own sales platform from 2019 and completed it in Q2 FY12/20. As part of the construction of its own logistics system, on September 7, 2020, the Company concluded basic transaction agreements for the buying and selling of pharmaceuticals with Suzuken Co., Ltd. <9987> and TOHO PHARMACEUTICAL CO., LTD. (a consolidated subsidiary of TOHO HOLDINGS CO., LTD. <8129>), and it became a general agent for these two companies.

For sales personnel, the Company has a structure of 57 personnel, comprising 51 MR personnel who have a high degree of expertise in the hematologic-disease field and six RSM (regional sales manager) personnel that it allocates to six blocks nationwide, while for marketing personnel, it has one KAM (KOL priority management manager) personnel and four HE (hematology expert) personnel, for a total of 62 personnel (of who, around 60% are contract personnel). Also, the sales personnel have completed training to enable them to respond quickly in the event of approval for the RTD formulation and an expansion of indications to relapsed and refractory DLBCL. In addition, the Company has completed the establishment of the in-house logistics and distribution system (one logistics base in each of east Japan and west Japan), the introduction of the mission-critical system, and the installation of the call center, so it has completed the preparations to enable a vertical launch after the end of the agreement in December 2020. For its current sales structure, the Company has been positively evaluated as having constructed a particularly highly specialized organization within the industry, and it is expected to contribute to increasing sales and improving the profit margin from 2021 onwards.

The loss in FY12/20 has been reduced from the initial forecast

2. Outlook for FY12/20

On September 17, the Company announced the revised FY12/20 results forecasts, of net sales to increase 7.2% YoY to ¥3,043mn, an operating loss of ¥4,592mn (compared to a loss of ¥4,301mn in the previous fiscal year), an ordinary loss of ¥4,656mn (a loss of ¥4,376mn), and a loss of ¥3,796mn (a loss of ¥4,376mn). Compared to the initial forecast, the net sales forecast has been downwardly revised, but the loss amounts have been reduced in the revised forecasts compared to the initial forecasts.

Outlook for FY12/20

(¥mn)

	FY12/19 Results	FY12/20 Company forecast	Change	% change	Rate of progress up to the Q2
Net sales	2,837	3,043	205	7.2%	44.7%
Gross profit	864	945	80	9.3%	34.9%
SG&A expenses	5,166	5,536	369	7.2%	39.2%
R&D expenses	2,441	2,260	-181	-7.4%	36.9%
Other SG&A expenses	2,724	3,276	551	20.2%	40.8%
Operating profit (loss)	-4,301	-4,592	-290	-	40.1%
Ordinary profit (loss)	-4,376	-4,656	-279	-	40.4%
Profit (loss)	-4,376	-3,796	580	-	49.7%

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

Net sales will be below the initial forecast due to the prolonging of the quality issue at the factory of the supplier, but the outlook is that due to the strengthening of the inspection system, sales will recover from ¥1,361mn in the H1 to ¥1,682mn in the H2. This is because sales of TREAKISYM® in Japan tend to be concentrated in the H2 due to seasonal demand, and also as in the H2, sales for overseas are expected to be comparatively firm, at around ¥200mn.

The rate of progress for the net sales forecast up to the Q2 was slightly low, at 44.7%. But due to the strengthening of the inspection system, net sales increased from ¥551mn in the Q1 to ¥809mn in the Q2 and are forecast to continue to recover from the Q3 onwards from conducting the same measure. Also, in the H2, sales for overseas are expected to be comparatively firm at around ¥200mn, and these are the grounds for thinking that the forecast can be achieved. However, it is highly likely that the gross profit margin will be below the initial forecast of 33.7%. This is because it was 24.2% in the H1, which is greatly below the average for the most recent three years of around 30%, due to strengthening of the inspection system, while in the H2 also, it is possible that the current inspection system will have to be maintained if the quality issue does not improve. The Company recognizes that the increase in costs due to the quality issue from the previous fiscal year to the H1 FY12/20 to be approximately ¥700mn, and it is currently considering what sort of compensation to seek from Astellas Pharma in the future.

On the other hand, in expenses, the rates of progress for the forecasts up to Q2 were 36.9% for R&D expenses and 40.8% for other SG&A expenses. In the H2, R&D expenses of ¥5mn are scheduled as a milestone payment from the marketing approval of the TRD formulation, which is the TREAKISYM® liquid formulation. But due to a change of development policy, the initially planned BCV clinical trial in Japan will not take place, so the Company reduced the forecast to ¥2,260mn, below the initial forecast of ¥2,731mn. Also, for SG&A expenses, it continues to work to keep down expenses, so it downwardly revised the forecast to ¥3,276mn, below the initial forecast of ¥3,504mn.

Moreover, in October 2017 the Company filed a petition with the International Chamber of Commerce against The Medicines Company for damages for the non-fulfillment of a licensing agreement for the post-operative*, self-administered pain-management medication SyB P-1501, and the ICC announced its arbitration ruling on July 2020. The content of the ruling was that while it did not recognize the Company's claim for damages, The Medicines Company was ordered to pay 50% of the Company's expenses relating to the arbitration proceedings, including attorneys' fees. Subsequently, the court issued its final ruling on September 1, ordering The Medicines Company to pay the Company US$4.95mn. With these expenses and contraction of R&D and SG&A expenses it downwardly revised the loss amounts compared to the initial forecasts.

* 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.

Is expected to start the exercise period for the 51st Stock Acquisition Rights at the appropriate time, while observing conditions

3. Financial condition

Looking at the financial condition at the end of the Q2 FY12/20, total assets were up ¥1,311mn on the end of the previous fiscal year to ¥6,585mn. The main change factors were that in current assets, accounts receivable-trade decreased ¥314mn and consumption taxes receivable declined ¥139mn, but due to the exercise of stock acquisition rights, cash and deposits increased ¥1,498mn, while merchandise and finished goods rose ¥151mn. In non-current assets, the total of software and software in progress increased ¥55mn, related to the construction of the Company's own sales platform.

Total liabilities were down ¥80mn on the end of the previous fiscal year to ¥792mn. The main change factors were that accounts payable-trade increased ¥72mn but accounts payable-other decreased ¥155mn. Net assets increased ¥1,392mn to ¥5,792mn, with the main factors being that the total of share capital and capital surplus increased ¥3,299mn following the exercise of stock acquisition rights, but retained earnings decreased ¥1,884mn due to the recording of a loss. As a result, the equity ratio rose from 71.7% at the end of the previous fiscal year to 78.9%.

The Company issued the 50th and 51st Stock Acquisition Rights (with Exercise Price Revision Clauses) via an allotment to EVO FUND on March 16 to raise business-activity funds (¥5,450mn) up to June 2021. Of these issues, the exercising of the 50th issue had all been completed by June, raising funds of ¥2,272mn. Funds of approximately ¥3.8bn were expected to be raised for the Company's existing pipeline development expenses and expenses to construct its own sales platform, but only around ¥2.2bn was raised as the stock price trended at a lower level than expected. The funds raised from the 51st Stock Acquisition Rights are expected to be around ¥1.6bn, which will be used for new licensing-in and funds for M&A, but because the amount raised in the 50th issue was less than expected, it is also possible that these funds will be allocated to development expenses and other expenses. The Company will determine the appropriate time period to start the exercising of the 51st Stock Acquisition Rights.

Balance sheet and management indicators

(¥mn)

	End-FY12/17	End-FY12/18	End-FY12/19	End-Q2 FY12/20	Change
Current assets	4,036	6,038	4,887	6,132	1,244
(Cash and deposits)	2,947	4,821	3,910	5,409	1,498
Non-current assets	215	200	386	452	66
Total assets	4,252	6,239	5,273	6,585	1,311
Total liabilities	1,012	1,337	873	792	-80
(Interest-bearing debt)	-	-	-	-	-
Net assets	3,239	4,901	4,400	5,792	1,392
Management indicator					
Equity ratio	63.6%	70.1%	71.7%	78.9%	7.2pt
Interest-bearing debt ratio	-	-	-	-	-

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 of 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). Cases in which an event triggering the extension of the commitment period occurs are excluded	In principle, commitment to the exercise of all Stock Acquisition Rights within 46 trading days from the exercise start date specified by the Company Cases in which an event triggering the extension of the commitment period occurs are excluded
Expected starting 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→2,272(Completed)	1,641

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

Mid-Range Plan

Targeting becoming profitable in FY12/21 and to continuously maintain an operating profit margin of at least 10%

1. Overview of the Mid-Range Plan

In the three-year Mid-Range Plan announced in February 2020, the plan is that the Company will achieve operating profit in FY12/21. The gross profit margin is expected to improve, because as previously stated, it will switch to its own sales platform from 2021, the sales price will rise and gross profit will increase, and also due to the switching from the TREAKISYM® lyophilized powder intravenous formulation to the RTD formulation. In addition, net sales will further increase from Q3 FY12/21 on the expansion of its indications to relapsed and refractory DLBCL, so the outlook for FY12/21 is for net sales of ¥9,008mn and operating profit of ¥1,031mn.

Mid-Range Plan

(¥mn)

	FY12/19 results	FY12/20 forecast	FY12/21 targets	FY12/22 targets
Net sales	2,837	3,043	9,008	10,816
Operating profit (loss)	-4,301	-4,592	1,031	1,482
Ordinary profit (loss)	-4,376	-4,656	987	1,438
Profit (loss)	-4,376	-3,796	1,356	1,717

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

The gross profit margin is expected to rise from the FY12/20 forecast of 33.7% to 70% to 80% in FY12/22 due to the shift to the Company's own sales platform and the progress made in switching to the RTD formulation. Also, SG&A expenses in FY12/20 are forecast to be ¥6,236mn, while from FY12/21 onwards also, they are expected to continue at around the ¥6bn level (excluding milestone payments). In this amount, R&D expenses will be recorded based on the anticipated development plans for TREAKISYM®, rigosertib, and BCV, and the forecast does not incorporate expenses such as initial agreement payments relating to new introductions into the pipeline. Other SG&A expenses are constituted mainly of expenses relating to TREAKISYM® sales and marketing operations, production and logistics operations, business development operations, and management operations. At the end of 2020, the Company plans to have 152 personnel, but it is not forecasting a significant increase in personnel after that. The reason profit will exceed ordinary profit from FY12/21 onwards is that, alongside becoming profitable, the progress made in eliminating the loss carried forward will be reflected in the tax effect accounting.

In FY12/22, it seems that the increase in sales for relapsed and refractory DLBCL will continue to contribute, and the Company is targeting net sales of ¥10,816mn and operating profit of ¥1,482mn. In the Mid-Range Plan, it has set yearly rolling targets and it plans to announce new three-year targets in February 2021. Within the targets, it is expected to make the BCV development schedule more concrete.

As previously stated, the Company is making progress as planned in building systems to become profitable, and the probability that it will become profitable is increasing. If considering risks, the TREAKISYM® drug price may be revised and the procurement of the RTD formulation may not progress as expected. But at FISCO, we think the likelihood of these situations occurring is low at the current time.

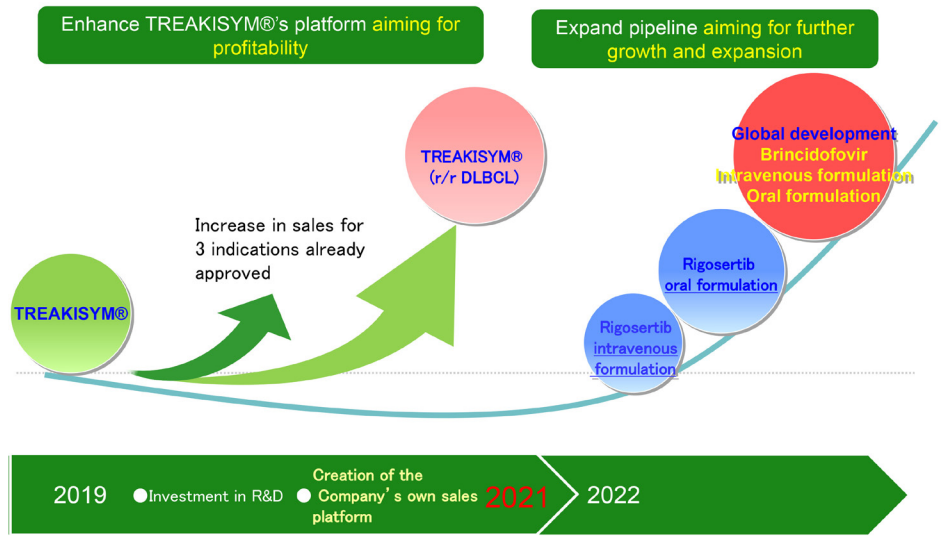
Sales growth potential will be even larger if the global roll-out of BCV proceeds well

2. 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. On the other hand, rigosertib did not achieve the primary endpoint in the results of the clinical trial indicated for high-risk MDS. Therefore, we must say that it has become less likely that the Company will be able to record sales for it in the next two or three years. However, it is possible that it will be developed indicated for other solid cancers.

Also, as the new pipeline, expectations are high for medium- to long-term growth from the addition of BCV. As previously explained, a feature of BCV is that it has high anti-virus activity against a wide range of DNA viruses, and if its development is a success globally as a viral-infection treatment after hematopoietic stem cell transplantation and organ transplantation, its sales have the potential to grow in the future to more than ¥100bn. So we shall be paying attention to developments going forward.

Illustration of "The 2nd SymBio"



Source: From the Company's results briefing material

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