RaQualia Pharma Inc.
4579 JASDAQ

5-Sept.-14

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It has enhanced its research infrastructure through industry-academia collaborations, and is expanding the scope of its research

RaQualia Pharma is a "drug discovery and development-type" biotech venture company that was established when the central research laboratory of Pfizer's Japanese subsidiary spun off from Pfizer. It utilizes the abundant drug discovery infrastructure that it inherited from Pfizer and its strengths in areas such as ion channels drug discovery technologies. It has a complete portfolio in the area of gastrointestinal diseases and is also advancing research and development in the pain area. Although ion channels drug discovery is a very difficult field, it is expected that innovative drug discoveries should be made in the area, and the company has concluded research collaboration agreements with multiple pharmaceutical companies and is searching for chemical compounds in this area.

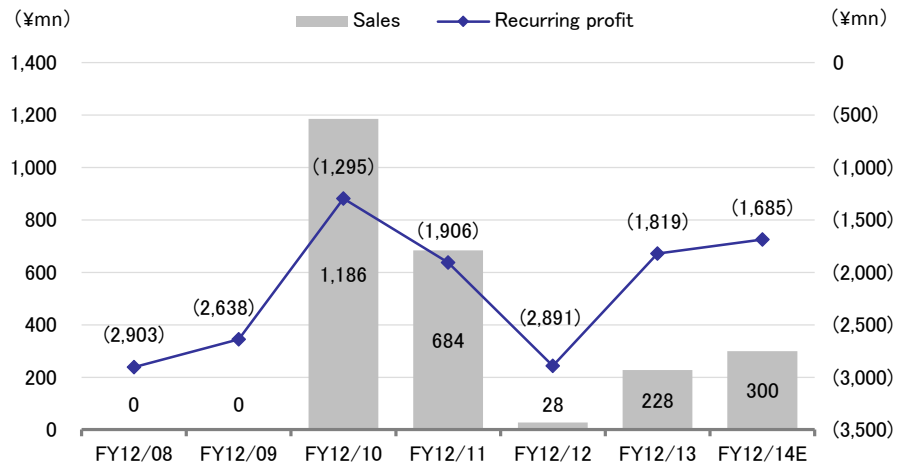
The company and the pharmaceutical companies to which it licenses its compounds are making steady progress in their current research and development activities. Progress in four major areas was confirmed for the first half of the fiscal period ending December 2014 (1H FY12/14). The company is smoothly advancing research and development in the areas of gastrointestinal disease, in which it has focused its efforts since the past, and pain. In addition, it has further enhanced its research infrastructure through industry-academia collaborations, and its policy is to further expand the scope of its research into the areas of cancer and immunologic diseases, and also transplant and regenerative medicine. A good example of this is the research collaboration agreement with Kyoto University Center for iPS Cell Research Application (CiRA) and iPS Academia Japan, Inc. Under this agreement, the company will search for compounds that will induce differentiation and proliferation of iPS cells into immune cells. Other than this, joint researches in the areas of cancer and immunologic disease using the identified compounds are underway by the company's subsidiary, AskAt Inc.

Currently, the company has made no changes to the framework of its medium-term management plan or its performance forecasts. Its first royalty income is forecast for FY12/16, and a medium-to-long-term scenario in which its performance stabilizes from FY12/17 onwards is expected. Even for its financing up to that point, it has basically achieved its targets for the current period. In the future, it is expected that it will steadily advance through the stages required for each of its drug discovery and development programs, and that a positive spiral will be created between its drug discovery and development activities and its fund raising activities.

Check Point

- A business model of generating earnings by discovering and licensing-out new drug compounds
- It is mainly making progress in four items among its programs for compounds it has already licensed-out
- On the whole, it is making steady progress in its medium-term management plan

Trends in full-year performance



Note: consolidated results from FY12/13

Company Outline

Its predecessor was the central research laboratory of the global pharmaceutical major Pfizer's Japanese subsidiary

(1) History

The predecessor to the company was the central research laboratory of the Japanese subsidiary of the global pharmaceutical major Pfizer, Inc., of the United States. This research laboratory served as Pfizer's exploratory research base and carried out drug discovery research, mainly in the areas of pain and gastrointestinal disease. However, in 2007 the decision was taken to close it.

Following the decision, the head of the laboratory at that time and some of its employees decided to conduct an employee buyout (EBO) and to continue as an independent drug discovery business, which resulted in the foundation of the company. It was founded in February 2008, and in July of the same year it launched its operations inheriting its human and material assets, its research and development portfolio, and the other businesses through the EBO. It listed on the Osaka Securities Exchange JASDAQ Growth Market in July 2011.

Company History

January 2007	Pfizer Inc. of the United States decides to close the central research laboratory of its Japanese subsidiary
February 2008	RaQualia Pharma Inc. is established
July 2008	It launches its operations utilizing the human and material assets and R&D portfolio it inherited from Pfizer
July 2011	It listed on the Osaka Securities Exchange's JASDAQ Growth Market
January 2013	It establishes its subsidiary, AskAt Inc.
February 2014	It concludes an agreement with Nagoya University to establish a division of analytical study on efficacy pharmacology as part of academic-industrial research collaboration It decides to transfer its drug discovery research functions to within Nagoya University
April 2014	It transfers its Efficiency Pharmacology Group to within Nagoya University
May 2014	It concludes a research collaboration agreement with Kyoto University Center for iPS Cell Research Application (CiRA) and iPS Academia Japan, Inc.
June 2014	It transfers its head office to Nakamura Ward, Nagoya (in front of Nagoya Station)

Source: prepared by FISCO from company materials

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A business model of generating earnings by discovering and licensing-out new drug compounds

(2) Business Model

The company is a research and development-type drug discovery company and it has a different business model than conventional pharmaceutical companies. In its business model, it generates earnings by licensing-out candidate compounds (licenses for technologies and patents) to the pharmaceutical companies that will be responsible for commercializing and selling them.

Drug research and development goes through three major stages; exploratory research to find candidate compounds, pre-clinical development to confirm its safety and efficacy in animal testing, and clinical development to assess its safety and efficacy in humans. After the developer applies for its regulatory approval as a new drug, the regulatory authorities approve it to be launched on the market. Clinical development is divided into three phrases; phase I, II and III. RaQualia Pharma's business area is from the exploratory research stage to phase II of clinical development.

RaQualia Pharma business area

Source: from the company's homepage

Moreover, the company actively collaborates with universities and other research facilities based upon its philosophy that "value for customers is created through an open collaborative network between academia (universities and research institutes) and companies." As a result of this philosophy, in this year alone it established a division for academic-industrial research collaboration in Nagoya University, is conducting joint research with CiRA, and initiated a doctor-initiated trial with Virginia Commonwealth University in the United States.

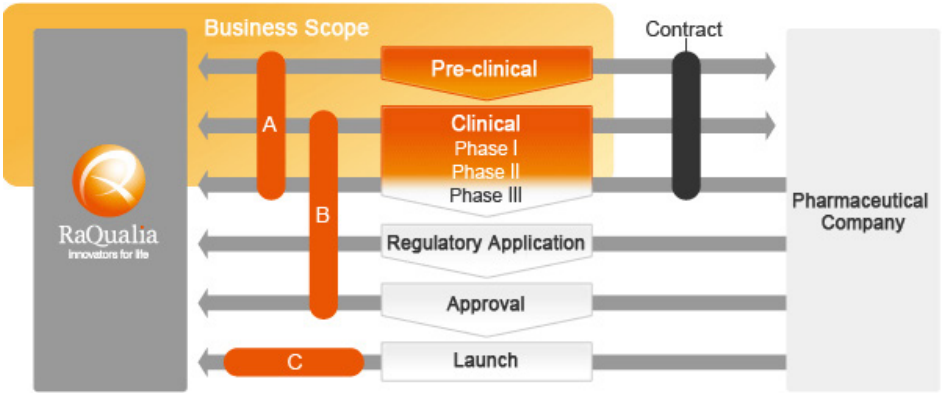
In terms of its sources of earnings, it has four main types, including earnings from lump sum payments from licensing agreements. The company is not a pharmaceutical company and therefore does not record drug sales. But in the event that a compound that the company has licensed-out is market launched as a drug, it receives royalty income of a fixed percentage of the drug's sales. The royalty rate (percentage of sales) is determined individually in each contract, but the industry norm is from 7% to 10%. Within its four types of earnings, royalties are expected to provide the company with the greatest amount. If we understand the company to be a so-called "fabless" type pharmaceutical company that specializes in research and development but does not fabricate anything, it becomes easier to get an image of its earnings structure.

RaQualia Pharma's sources of earnings

License agreement lump-sum income	Income received when its licenses-out a development to a pharmaceutical company that will undertake its development
Milestone income	Income received when milestones are reached as the clinical trials are progressed after licensing
Royalty income	Income received as a fixed percentage of sales after a product is market launch
Research collaboration income	In accordance with the conditions of the research collaboration agreement, income received as recompense for the research findings provided by the company up to that time on the start of the collaborative research, and also income received as recompense for the work provided during the collaborative research period

Source: prepared by FISCO based on the RaQualia Pharma homepage

RaQualia Pharma's earnings model



Source: reproduced from the company's homepage

The company's strength is in ion channels drug discovery technologies that are highly complex and so have high barriers to entry

(3) RaQualia Pharma's characteristics and strengths

The company has two main strengths. The first is its technologies for ion channels drug discovery. While ion channels drug discovery is very difficult and the barriers to entry are high, potent drugs with a wide range of pharmacological effects are expected in this area Therefore, should such a drug be commercialized, the product is expected to be highly marketable and to bring about a new age of drug discovery. Its second strength is that it has a complete infrastructure for drug discovery with a library of about 380,000 compounds and expertise in screening robots and analysis.

(a) A competitive advantage in ion channels drug discovery

One method of classifying drugs is to focus on the drug's mechanism of action. A drug has an effect because it acts on some location in the body, and this location is known as the site of action. The site of action can be broadly categorized into receptors, enzymes, ion channels, and nuclear receptors and others. In the cells that make up the human body, the ion channels are the proteins that play a role in helping ion material (such as potassium ion and sodium ion) penetrate cell membranes, and ion channels drug discovery signifies the development of drugs whose site of action is an ion channel.

Ion channels drug discovery signifies a new generation of drugs. Ion channels have selectivity as each ion channel limits substances to pass through. They are known as the potassium channel or the sodium channel. Skillfully utilizing this selectivity makes possible new drugs with a different approach to the current therapies, such as drugs that act strongly on a specific location or disease. In terms of the indicated treatment area for these drugs, effective new drugs are expected for pain and for circulatory organs (the heart-disease field), urinary, and gastrointestinal conditions. But on the other hand, there are issues to resolve, as drug discovery technologies in this area are very complex, the question remains of how to separate the side effects, and also it is difficult to make the drug discovery process more efficient. For these reasons, it is not easy for even major pharmaceutical companies to enter this field.

While the company faces various hurdles to clear for its ion channels drug discovery, it also possesses solutions to these problems such as the abundant library of 380,000 compounds, utilization of screening robots to increase their efficacy, the joint research with universities and government research institutes, and its own expertise in refining and analysis. These factors serve as competitive advantage over other companies in the same business.

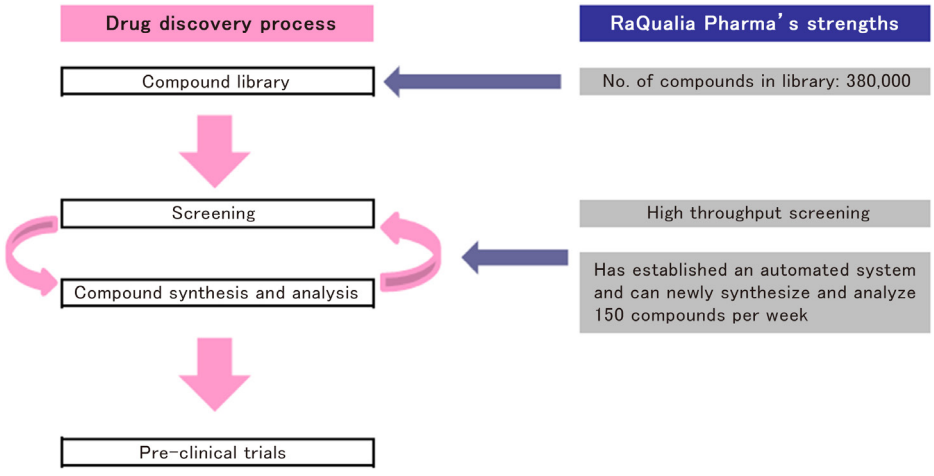
Problems for ion channels drug discovery and RaQualia Pharma's competitive advantage

Problems for ion channels drug discovery	RaQualia Pharma's competitive advantage / solutions
Many unknowns: a comparatively new field and therefore there are many unknowns about its participation in physiological functions and business form	Is conducting collaborative research with universities, public research institutes, and pharmaceutical companies
Conventional methods do not apply: as natural ligands (note) do not exist, naturally biologically active substances are not involved	It has established an automated system for refining and analyzing its abundant chemical compounds library
Screening difficulties: existing high throughput screening methods are limited to indirect observations and a complex measurement system measuring living cells is required	It has jointly developed a device for measuring activity in ion channels with Hamamatsu Photonics

Note: ligands are materials that differentially bind to specific receptors
Source: prepared by FISCO from company materials

The company's strengths through the drug discovery process can be illustrated as the figure below. It starts by using robots to select from many basic compounds (the compounds library) a compound that has the potential to become a class of drug in the targeted area. After that, it synthesizes and analyzes it repeatedly with the aim of discovering a compound that will be effective as a development candidate. During this process, a trial and error approach is repeated and the key point here becomes how quickly and accurately can the enormous quantity of work that is required be completed.

The drug discovery process and RaQualia Pharma's strengths and characteristics



Source: prepared by FISCO

The company has introduced high throughput screening (HTS) robots into the process of searching for and selecting compounds and in this area, it possesses the same capabilities as other major drug companies. But there are many areas in ion channels drug discovery where HTS cannot be applied, and in these areas manual analyses become essential. In order to increase the efficiency of the research and development for these areas, the company has jointly developed with Hamamatsu Photonics<6965> a device for measuring activity in ion channels. Compared to work manually done, this device has succeeded in improving efficiency by around 10 times. Further, even when the analysis is manually done in the company, it has installed a laboratory solely for this purpose and employs a number of researchers with advanced skills who specialize in evaluating ion channels, which is enabling it to increase the precision of its evaluations.

It cannot be denied that there exists a major gap between the company and the major pharmaceutical companies in terms of the number of staff it employs for its research and development and also its budget for research and development. In order to bridge this gap, the company has focused its drug discovery on two areas; gastrointestinal conditions and pain. Further details are provided below, but up to the present time it has been steadily developing new drugs in both these fields, while from 1H FY12/14, it has expanded its development into the areas of immunization and cancer.

(b) Competitive advantage in the ion channels drug market

At the present time there are not many ion channels drugs on the market as they belong to a new generation of drugs. Of the 483 classes of drugs, when categorized by mechanism of action, the majority are either receptor or enzyme drugs, with ion channels-type drugs constituting no more than 5% of all drugs in numerical terms. But based on sales, they constitute 22%. It is possible to infer from this that while ion channels drugs are difficult to develop and only a small number have been marketed, once they are launched they could record strong sales as drugs that are highly effective and in many cases grow into being medium- or large-scale drugs.

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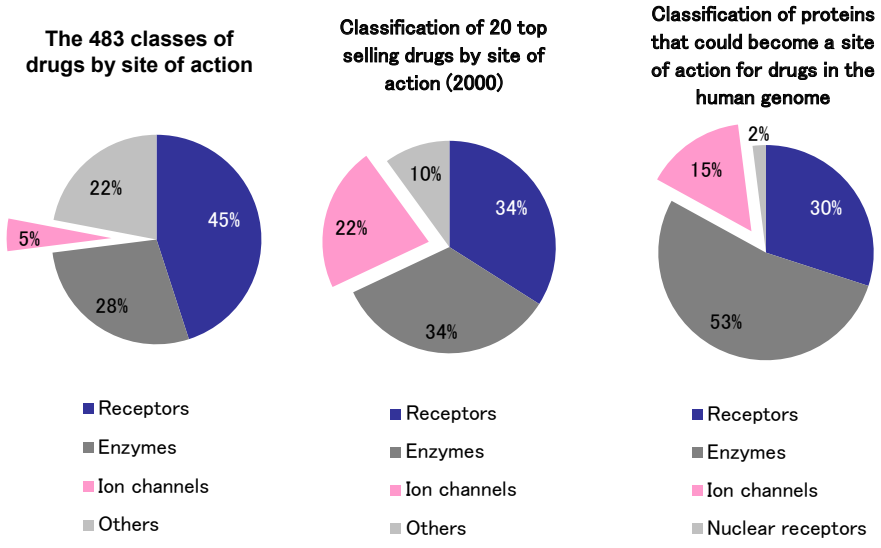


Source: Medium-term management plan briefing materials (source: Drews J. Science (2000))
Medium-term management plan briefing materials (source: Yoshida T et al. FPJ (2005))
Homepage of the Graduate School and Faculty of Pharmaceutical Sciences, Kyoto University (source: TIPS22, 23(2001))

■ Status of the company's portfolio of licensed compounds

It is mainly making progress in four items among its programs for the compounds it has already licensed-out

At the current point in time, the company has licensed-out five compounds for human drugs and two compounds for animal drugs to pharmaceutical companies. In 1H FY12/14, the main progress was made for its acid pump antagonist (RQ-4), 5-HT4 partial agonist (RQ-10), EP4 antagonist (RQ-7, animal drug), and Dalbavancin.

Portfolio of licensed compounds

	Program	Compound Code	Main indication	Licensee	Agreement region	Exploratory research - pre-clinical	Clinical trial P-I	Clinical trial P-II	Clinical trial P-III	Comments
Human drugs	Ziprasidone	RQ-00000003	Schizophrenia/ bipolar disorder	Meiji Seika Pharma	Japan				Preparations underway	Already on sale in Europe and the United States
	Dalbavancin	RQ-00000002	MRSA	Durata Therapeutics	Japan					In the U.S., was approved by the FDA in the U.S. in May and launched in July. P-I scheduled for Japan in the future
	Acid pump antagonist	RQ-00000004	Gastro-esophageal reflux disease	CJ Healthcare	South Korea, China, Taiwan			Underway (South Korea)		Progressing steadily
		RQ-00000774		-	Japan, global		Begun			P-1 have begun in Japan (June). Also, its global development is scheduled.
	5-HT4 partial agonist	RQ-00000010	Gastroparesis, functional GI disorder	CJ Healthcare	Taiwan, India, and East Asia		Being planned			
				-	Japan, global			Being investigated		Launched a doctor-initiated clinical trial for Parkinson's disease patients at Virginia Commonwealth University in the U.S. Investigating conducting a global P-II in-company
	EP4 antagonist	RQ-00000007	chronic inflammatory pain, acute pain	Maruishi Pharmaceutical	Japan, South Korea, China, Taiwan		Planning			Injection only (AskAt Inc. is investigating an oral version)

	Program	Compound Code	Main indication	Licensee	Agreement region	Exploratory research - pre-clinical	pre-clinical trial Dosage exploration	pre-clinical trial large scale	Market launch	Comments
Animal drugs	Ghrelin receptor agonist (RQ-5)	RQ-00000005	Loss of appetite / weight loss	Aratana Therapeutics Inc.	Global			Underway		Scheduled for completion end of 2015 and market launch in 2016
	EP4 antagonist	RQ-00000007	Arthrosis deformation					Begun		Large scale clinical trial was launched in May. If it proceeds as planned, market launch is planned for 2017.

Note: The boxes highlighted in red are the items for which progress was mainly made in 1H FY12/14
Source: prepared by FISCO from company materials

(1) Detail of the programs for which progress was mainly made in the past half year

Acid pump antagonist (RQ-4)

RQ-4 is an acid pump antagonist indicated for gastro-esophageal reflux disease (GERD) and as a next-generation new drug, it is expected to replace the proton pump inhibitors that are currently the mainstream treatment (including Pariet from Eisai <4523> and Takepron from Takeda Pharmaceutical <4502>).Takeda Pharmaceutical is at the head of the field in the development of an acid pump antagonist and has applied for approval for Vonoprazan (TAK-438), while RaQualia Pharma's RQ-4 is next in line. The company began a phase I trial of RQ-4 in June 2014 in Japan. This phase I trial is scheduled to be completed within the year and the company is searching for licensees for the Japanese market in conjunction with implementing this phase I trial.

TAK-438 is currently awaiting regulatory approval and it is expected to receive approval within Japan during FY03/15. If TAK-438 is approved, a development chain of H2 blocker→proton pump inhibitor→acid pump antagonist will be established, which is expected to prove advantageous for RaQualia Pharma's licensing-out of RQ-4, which is currently running in second place.

The scale of the market the company is targeting with RQ-4 is large and the global market for proton pump inhibitors is said to be worth around ¥2 trillion. Presently, the company is planning to license it out in the Japanese market, so ultimately the greatest expectations are for this market. At the current point in time, it is difficult to judge what the scale of the company's royalty income will be. But of reference are the sales totals in Japan in fiscal 2013 of the main proton pump inhibitors, which were ¥67.6 billion yen for Takepron from Takeda Pharmaceutical, ¥54.2 billion for Nexium from Daiichi Sankyo <4568>, and ¥47.3 billion for Pariet from Eisai.

In the markets of South Korea, Taiwan, and China, RQ-4 has been licensed to CJ Health Care Corporation (subsequently, CJ Corporation), which is currently carrying out the phase II trial. This trial is also scheduled to be completed within the year, and it is expected to progress to the phase III trial at the start of 2015. CJ Corporation also announced in July 2014 that RQ-4 has been selected for South Korea's "new drug development issues" project. This is a national project for new drug development that will see the investment of a total of 1.06 trillion won (530 billion won by the government and 530 billion won by the private sector) by 2020. CJ Corporation has also discussed advancing into the China market for RQ-4 thanks to its selection for the new drug development project.

However, in the South Korea's pharmaceutical market, it takes two years averagely from the time of application for a new drug to obtain approval, which is double the time it takes in Japan and the United States. From the perspective of market scale, South Korea's market is significantly smaller than Japan's. At the earliest, CJ Corporation will launch RQ-4 as a new drug in 2018 and it should achieve annual sales on the scale of ¥5 billion. It is thought that RaQualia Pharma's royalty income would be around 7% to 10% of that. We expect that CJ Corporation will complete its clinical trials quickly and expand the development area for this drug.

Development status of RQ-4 and its main schedule

March 2013	Granted substance patent in the U.S. (patent is recognized)
May 2013	CJ CheilJedang (currently, CJ Healthcare) begins phase II trials in South Korea
October 2013	Granted usage patent in the U.S.
June 2014	RaQualia Pharma begins phase I trials in Japan
End of 2014	CJ Healthcare completes phase II
Start of 2015	CJ Healthcare begins phase III trials
End of 2015	RaQualia Pharma completes phase I trials in Japan around this time
2016	CJ Healthcare applies for regulatory approval for the new drug
2018	CJ Healthcare market launches the new drug

Source: prepared by FISCO based on company materials and interview

5-HT4 partial agonist (RQ-10)

RQ-10 is a compound indicated for conditions including gastroesophageal reflux, gastroparesis, and functional GI disorder. This drug targets one of the serotonin receptors (5-HT4) and is expected to have extremely strong efficacy and high levels of safety compared to Mosapride, which has already been launched under the brand name of Gasmotin by Sumitomo Dainippon Pharma <4506>. The company is targeting the East Asia market, particularly South Korea, and it has licensed it out to CJ Corporation of South Korea. But it is also aiming to license it out to other regions and RaQualia Pharma itself is conducting clinical trials. Currently, it has completed phase I trials in the United Kingdom (May 2013) and is preparing for phase II trials.

In terms of other progress made in 1H FY12/14, the company and the Virginia Commonwealth University in the United States launched a doctor-initiated clinical trial for Parkinson's disease patients. As previously stated, RQ-10 has completed its phase I trial, in which it clearly promoted gastric emptying in extremely small doses (3 μg/body) in healthy adults. Moreover, it was confirmed that there are no safety issues even at a dose 1,000 times greater than the dose for which the gastric emptying effect was observed.

The company is aiming to license out RQ-10 at an early stage and is planning on initiation of the phase II trial during 2014. If its effectiveness is verified for the treatment of Parkinson's disease under the current research agreement, that will reinforce the company's licensing activity of RQ-10.

It is too soon to refer to how RQ-10 will contribute to earnings. In the Japanese market in fiscal 2013, Sumitomo Dainippon Pharma's Gasmotin recorded sales of ¥15 billion, which might be used for reference when estimating what the sales of RQ-10 will be. CJ Corporation estimates sales in South Korea will be between ¥2 billion and ¥3 billion.

Development status of RQ-10 and the main schedule

May 2013	RaQualia Pharma begins phase I trials in the United Kingdom
May 2014	Launches a doctor-initiated clinical trial for Parkinson's disease patients at Virginia Commonwealth University in the U.S
2014	RaQualia Pharma investigates beginning phase II trials globally
2014	CJ Healthcare prepares to begin phase I trials in South Korea

Source: prepared by FISCO based on company materials and interview

EP4 antagonist (RQ-7, animal drugs)

EP4 antagonist is an animal drug indicated mainly for osteoarthritis in dogs and cats. Targeting a worldwide market, the company has already licensed it out to Aratana Therapeutics Inc., (subsequently, Aratana) and in May this company began a large-scale clinical trial on dogs. This is comparable to a phase III trial for humans. The time period of this large-scale clinical trial is expected to be around two years, and at the current point in time it is set to finish around 2016, at which time a new-drug application (NDA) will be made. If its progress is the same as a typical case, it will be launched at the end of 2017.

As an animal drug its market scale is comparatively large and annual sales on a scale of ¥10 billion are expected for each of North America and Europe. Even though it is an animal drug, the royalty structure is the same as for a human drug and if it becomes a product recording sales of ¥20 billion a year, the company will collect around 7% to 10% of that as its royalty income (Please note that this royalty rate is based only on typical cases in the same industry and is not based on the company's actual agreement).

Dalbavancin

Dalbavancin is an antibiotic indicated for Methicillin-Resistant Staphylococcus Aureus (MRSA), and is a drug that was originally developed by Pfizer. Pfizer licenses it out to Durata Therapeutics Inc. (subsequently, Durata) of the United States for the Europe and U.S. markets. RaQualia Pharma had the rights to it for the Japanese market, and it transferred them to Durata. Based on the approval of Dalbavancin by the FDA in the United States in May 2014, Durata market launched the drug in the United States in July. As Pfizer is the licenser in the U.S. market, this had no direct effect on RaQualia Pharma's earnings. However, the fact that it has been approved and launched in the United States is expected to add momentum to the company's clinical trials and search for licensees in Japan. Due to the rules for clinical trials, the phase I trial has to be completed in Japan but subsequently it may be possible to use the data from clinical trials conducted overseas to advance directly through to the phase III trial. In this scenario, at the very earliest it can be expected to be market launched in Japan during 2018.

The goal is for Dalbavancin to be a replacement drug for Vancomycin. Vancomycin must be taken twice a day, but Dalbavancin has a long half-life (it maintains its medicinal effect over a long period of time) and so it has the advantage that it only needs to be taken once per week. There are great expectations for Dalbavancin in the United States due to the emergence of Vancomycin-Resistant Staphylococcus Aureus (VRSA), but presently VRSA has not been confirmed in Japan. Essentially, the medical conditions that Dalbavancin is indicated for are limited, so there should not be excessive expectations for it. Annual sales within Japan in the range of ¥2 billion to ¥3 billion are forecast. But it is possible that its sales will exceed this amount if the conditions that Dalbavancin is indicated for are expanded to include, for example, pneumonia and septicemia.

It is making steady progress in its Ghrelin receptor agonist that should provide it with royalty income the soonest among all its compounds

(2) Status of other drugs in the pipeline

Ziprasidone

Ziprasidone is a drug indicated for schizophrenia and bipolar disorder and is already being sold by Pfizer in at least 76 countries and regions, including the United States. RaQualia Pharma acquired the rights for it in Japan from Pfizer and has licensed it out to Meiji Seika Pharma, Co., Ltd. Meiji Seika Pharma has currently completed the phase II trial within Japan and is preparing to begin the phase III trial. If smooth progress is achieved up to its market launch, then when considering the sales of existing, similar drugs, it could grow into a drug with annual sales of more than ¥10 billion.

EP4 antagonist (RQ-7, human drug)

EP4 antagonist is a drug for humans indicated mainly for chronic inflammatory pain and acute pain, and the injection version has already been licensed-out to Maruishi Pharmaceutical for the Japan and East Asia markets. Maruishi Pharmaceutical is at the stage of planning the phase I trial within Japan. As it is limited to an injection, even if it is market launched in the future its sales may not be all that high. AskAt Inc. is conducting clinical trials for an oral version of this drug. RaQualia Pharma has licensed the drug to AskAt Inc. and if the oral version is market launched, it will receive a fixed percentage of the earnings from it in the future.

Ghrelin receptor agonist (RQ-5)

The ghrelin receptor agonist (RQ-5) is indicated for anorexia and weight loss in cats and dogs, and the same as with RQ-7, the company has licensed it out to Aratana of the United States for the worldwide market. Aratana is currently conducting a large-scale clinical trial for RQ-5 and is making steady progress. At the current point in time, Aratana is planning a NDA for RQ-5 after the end of 2015. It expects to receive approval approximately one year later and to launch it around the end of 2016. The market scale for similar drugs is thought to exceed ¥10 billion in both North America and Europe. Among all of the company's compounds, RQ-5 is positioned as the one that will provide it with royalty income the soonest.

■ Status of licensing-out candidates in the pipeline

It plans to develop worldwide licensing activities for its 5-HT2B antagonist

The table below shows the research and development being conducted by the company and its subsidiary AskAt Inc. with the goal of licensing-out compounds in the future. The development stages in 1H FY12/14 are generally the same as they were previously and the company seems to be steadily advancing its research and development.

5-HT2B antagonist

5-HT2B is one type of gastrointestinal hormone serotonin (5-HT) and this compound has the medicinal effect of suppressing the activity of 5-HT2B, which is expected to reduce visceral pain and normalize gastrointestinal motility. The joint research conducted with Gunma University has indicated that the compound controls abnormal defecation, while it does not have an excessive effect on normal intestines, and therefore it is expected to be a new therapy for irritable bowel syndrome (IBS). The safety trial and pharmacokinetic trial were completed in 2013 and its pharmacological trial is scheduled to be completed in 2014.

On August 20th, it was announced that the company received an allowance for a substance patent for 5-HT2B antagonist in Japan (application number 2010-539267) that had been under review. This allowance will strengthen the company's intellectual property rights in Japan in the future, following the previously-granted patents in the United States and other regions, and it plans to continue to focus on obtaining patent rights in other countries and developing its licensing activities worldwide, including in Japan.

Motilin receptor agonist

Motilin receptor agonist is a compound that acts on and has a medicinal effect on Motilin, which is one of the gastrointestinal hormones, and it has been confirmed that it is highly effective for activating motility in cases of gastric motor dysfunction. The compound is expected to improve conditions including gastroparesis, postoperative ileus, functional dyspepsia, and other related diseases. Currently there is no Motilin receptor agonist that has received manufacture and sales approval. Therefore, should the company's compound be market launched, it may become a ground-breaking new drug. It plans to continue pre-clinical trials in 2014.

■ Status of licensing-out candidates in the pipeline

Status of programs that are candidates for licensing out

	Program	Compound code	Indication	Exploratory research	Pre-clinical trial	Clinical trial		
						P-I	P-II	P-III
RaQualia Pharma	Selective sodium channel blocker	-	Pain	Underway				
	TRMPM8 blocker	-	Pain	Underway				
	5-HT2B antagonist	RQ-00310941	Irritable bowel syndrome		Report being prepared			
	Motilin receptor agonist	RQ-00201894	Gastroparesis		Report being prepared			
	Program	Compound code	Indication	Exploratory research	Pre-clinical trial	Clinical trial		
						P-I	P-II	P-III
AskAt Inc.	EP4 antagonist	AAT-007	Arthrosis deformation				Underway	
			Autoimmune diseases, Allergies			Underway		
			Cancer	Underway				
		AAT-008	Arthrosis deformation			Underway		
			Autoimmune diseases, Allergies			Underway		
			Cancer	Underway				
	COX-2 inhibitor	RQ-00317076	Acute pain				Underway	
	5-HT4 partial agonist	RQ-00000009	Central nervous system diseases			Underway		

Source: prepared by FISCO from company materials

In addition to research and development, the company strengthens protection of its intellectual property and is strategically securing patents for its compounds. It has assigned staff and budget on an exceptional scale as a venture company. A critical factor for drug discovery venture company is acquiring patents that can exclude other companies over a wide area, and therefore the company is not simply acquiring substance patents, it is also aiming to acquire a range of peripheral patents and to construct a portfolio consisting of high value intellectual property.

Recent measures to strengthening intellectual property

2013	
February 22	Granted patent in Europe for EP4 antagonist
March 25	Granted patent in the United States for acid pump antagonist
July 24	Granted patent in China for EP4 antagonist
October 10	Granted patent (usage patent) in the United States for acid pump antagonist
2014	
January 7	Granted patent in the United States for a motilin receptor agonist
February 6	Granted patent in the United States for a 5-HT4 receptor partial agonist
May 12	Granted patent in China for a motilin receptor agonist
May 28	Granted patent in Japan for a selective sodium channel blocker
August 20	Granted patent in Japan for a 5-HT2B antagonist

Source: prepared by FISCO from company materials

■ Joint research

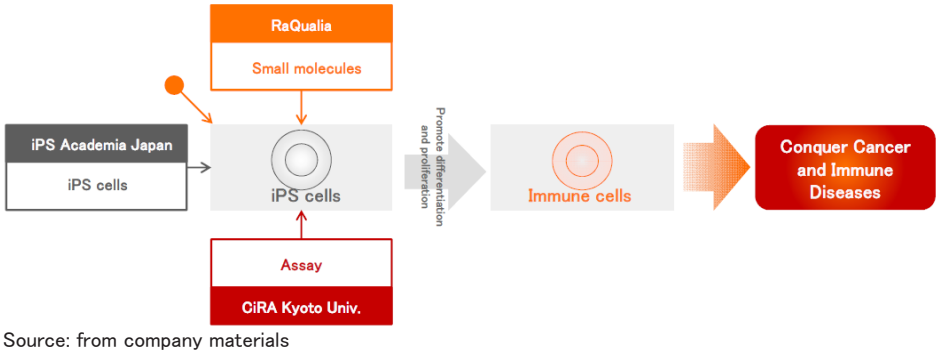
It has concluded a research collaboration agreement with CiRA of Kyoto University and iPS Academia Japan

(1) Joint research with CiRA and iPS Academia Japan

In May 2014, the company concluded a research collaboration agreement with the Department of Cell Growth and Differentiation in Kyoto University (subsequently, CiRA) and iPS Academia Japan for the differentiation and proliferation of iPS cells.

■ Joint research

Joint research with CiRA, and the iPS Academia Japan



Source: from company materials

The objective of this joint research is to search for promising small molecular compounds that can effectively induce differentiation and proliferation of iPS cells into immune cells. The company will select small molecular compounds from its own library of 380,000 compounds and deliver them to CiRA. iPS Academia Japan will provide the iPS cells and CiRA will carry out the assay.

The goal of this joint research is to develop a technology that efficiently makes immune cells from iPS cells, and the small molecular compounds that the company discovers will function as the catalyst. There are high hopes that immune cells made from iPS cells will play a role in defeating cancer and various immunologic diseases.

The company's research and development capabilities will be further enhanced by applying its inherent strengths of screening and optimizing small molecular compounds to this new field. Also, there are still many unknowns about the role small molecular compounds in iPS cell-related technologies. Therefore, there will be increased interest in the research findings from this project in the future.

It is impossible to estimate what its contribution to earnings will be at the current stage. However, if the company obtains the rights as the inventor, it is possible that it will be able to commercialize the technologies resulting from this research and thereby generate earnings.

In collaboration with the Nagoya Institute of Technology, it is aiming to reduce by half the cost of manufacturing AIDS drug

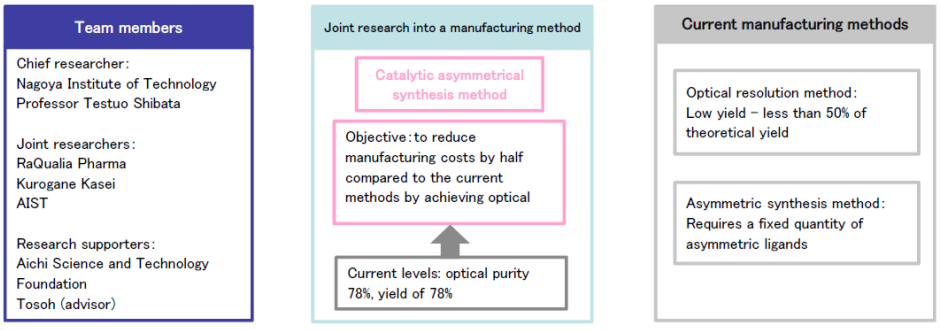
(2) Joint research with Nagoya Institute of Technology

According to the United Nations, there were 35.3 million HIV positive people in the world at the end of 2012, and among them 2.3 million people were newly HIV positive. But only 37% of these people were receiving treatment through antiviral drugs. The background to this is that AIDS drugs continue to be expensive and so they are not being used sufficiently in developing countries. One of the factors behind the high prices of AIDS drugs is the difficulty in synthesizing compounds in the manufacturing process.

In the joint research that it is advancing with the Nagoya Institute of Technology, the company is developing a catalytic asymmetrical synthesis method to replace the current manufacturing methods (optical resolution method and asymmetric synthesis method). Its goal is to reduce manufacturing costs by half compared to these current methods. In order to achieve this goal, it must realize optical purity of 95% or more and yield of 90% or more, compared to the current levels of 78% and 78% respectively. It will not be easy to raise the optical purity and yield levels up to these targets and we must wait to see what progress it achieves in its research and development in the future.

The contribution to earnings from this joint research has not been estimated at all at the current time, including in the medium-term management plan. In terms of the business model, it is expected that the company will acquire the patent for the manufacturing method, allow pharmaceutical companies to use it, and obtain royalty income as a fixed percentage of sales.

Outline of the joint research with Nagoya Institute of Technology



Source: prepared by FISCO from company materials

Its ion channels drug discovery technologies are attractive to major pharmaceutical companies

(3) Joint research with corporates

The company's technologies for ion channels drug discovery are attractive to major pharmaceutical companies. Currently, the company has concluded research collaboration agreements with two companies, Ajinomoto Pharmaceuticals and Asahi Kasei Pharma, relating to ion channels drug discovery, and is conducting joint research with them in order to discover compounds for clinical development. It is also conducting research with Interprotein Corporation into candidate compounds for clinical development targeting interaction between specific proteins, and with Carna Biosciences, Inc. <4572> to discover compounds as candidates for clinical development targeting specific kinase. Its research collaboration agreement with Eli Lilly of the United States ended in June 2014.

In general, joint research takes place primarily at the initial stage of a drug's development. However, in many cases, the terms for milestone payments and royalty income in the event that the product is market launched are referred to at the stage of concluding the research collaborative agreement. The company's research collaboration agreements also include such details and if it can steadily discover compounds that are candidates for clinical development, it can be expected to receive milestone payments as it progresses their development through the pre-clinical trial and clinical trial stages.

A list of the company's joint research projects

Name of joint research company	Target	Compound	Indication	Search	Pre-clinical trial	Clinical trial			Agreement description	Date agreement concluded
						P-I	P-II	P-III		
Ajinomoto Pharmaceuticals	Ion channels	Being identified	Gastrointestinal	●					Joint research targeting specific ion channels for gastrointestinal indications	October 2012
Asahi Kasei Pharma	Ion channels	Being identified	Undisclosed	●					Joint research targeting specific ion channels (indication undisclosed)	November 2013
Interprotein Corporation	Interaction between specific proteins	Being identified	Pain	●					Joint research targeting interaction between specific proteins for pain indications	February 2013
Carna Biosciences	Specific kinase	Being specified	Undisclosed	●					Drug discovery research targeting specific kinase	March 2013

Source: prepared by FISCO from company materials

■ Medium-term management plan and performance forecasts

On the whole, it is making steady progress in its medium-term management plan

(1) Outline of the medium-term management plan

The company announced its three-year medium-term management plan for the period running from fiscal 2014 to fiscal 2016 on February 28, 2014, and has not changed its forecasts up to the present time.

In terms of its progress during the past half year, the company has steadily implemented its medium-term management plan for those items under its own control. The examples of these achievements are: its conclusion of a research collaboration agreement with CiRA and iPS Academia Japan, its launch of phase I clinical trials of RQ-4 in Japan, its transfer of its research and development base to Nagoya University, its recording of a gain from sales of investment securities, and its issue of share options. Other aspects of the company's activities are also progressing steadily, such as the development and promotion of its license programs and its acquisition of licensees for its existing pipeline. For example, progress in this area includes the launch of Dalbavancin by Durata of the U.S. and the start of a large-scale clinical trial of an animal drug (RQ-7) by Aratana.

Outline of the medium-term management plan

Basic Policy	Development / execution/ accomplishment of business plans with a high probability of improving corporate value		
	Presentation of specific policy/measures toward steadily earning income		
Outline of the medium-term management plan	Outline	Content	Basic actions
	Enhancement and improvement of the research and development portfolio	Acceleration of drug discovery research through industry-academia collaboration	Establish an industry-academia collaborative research division in Nagoya University
		Creation of new development compounds by collaborative research with external agencies, etc.	Ajinomoto Pharmaceutical, Asahi Kasei Pharma
		Continuous creation of new development compounds by the company's own evaluation system	
	Improvement of the monetization of research and development results	Obtain milestone and royalty income that are expected in the mid- and long-term by strengthening alliance management	CJ Healthcare, Meiji Seika Pharma, Maruishi Pharmaceutical, Maruishi Pharmaceutical, Aratana Inc., etc.
		Promotion of licensee programs through the program value improvement and acquisition of revenue	RQ-10, RQ-4, Motilin receptor, 5-HT2B, etc.
		Monetization of the results of research by industry-academia collaboration	
	Compression of business costs by concentrating management resources	Improvement of development stages by utilizing own funds for core programs and external project finances	Currently implementing P-I in Japan for RQ-4
		Compression of fixed costs (facility-related costs) by gradually transferring the research and development center	Transfer R&D base to Nagoya University
		Continuously reviewing and reducing fixed costs	Transfer headquarters
	Management stabilization and business continuity	Consideration and execution of fund raising to get through the so-called "Death Valley" until it is steadily acquiring income	Record gain on sales of investment securities for sales of shares in Aratana Inc and execute share acquisition rights
		Promotion of strategic capital (business) alliances	
		Consideration and execution of merit-based incentives for employees	Allocate stock options to employees

Source: prepared by FISCO from company materials

Under the above basic policies, the company produced the performance forecasts shown below, assuming only those programs that are highly likely to generate earnings. During the period of the current medium-term management plan, lump sum payments and milestone income from licensing agreements are its main sources of earnings, and as the earnings from these businesses are still at a low level, it is forecast to continue to record an operating loss and net loss. However, it plans to decrease its businesses expenses at the same time as it increases its business earnings, and it is steadily implementing a range of measures toward achieving profitability soon after the end of the current medium-term management plan.

Performance targets in the medium-term management plan

(unit: ¥mn)

	Operating Revenue	Operating Cost	Operating Loss	Ordinary Loss	Current Term Net Profit or Loss
FY2013 (Result)	228	2,366	(2,137)	(1,819)	(1,108)
FY 2014 (Plan)	300	1,984	(1,684)	(1,685)	(282)
FY 2015 (Target)	600	1,698	(1,098)	(1,100)	(1,155)
FY 2016 (Target)	1,200	1,686	(486)	(485)	(489)

Source: prepared by FISCO from company materials

The main sources of earnings during the current medium-term management plan are expected to be lump sum payments and milestone income from licensing agreements

(2) Details of its earnings plan

The table below shows the breakdown by program and by source of earnings during the current medium-term management plan. The contents of this table remain unchanged from the last report. As was previously stated, the main sources of earnings during the period of the current medium-term management plan are lump sum payments and milestone income from its licensing agreements. In FY12/16, which is the final year of the medium-term management plan, the company will obtain royalty income from the market launch by Aratana of an animal drug (ghrelin receptor agonist, RQ-5) that improves anorexia and weight loss in animals. The total market scale in Europe and the United States for similar drugs is thought to be around ¥20 billion, but as its launch will occur around the end of the year, the bulk of the earnings in the FY12/16 are expected to be provided by milestone income from another drug that is being developed by Aratana (EP4 antagonist, RQ-7).

Breakdown by program and by source of earnings during the current medium-term management plan (¥ mn)

(unit: ¥mn)

Sources of earnings	FY2013 Result		FY2014 Plan		FY2015 Target		FY2016 Target	
	Amount	Content	Amount	Content	Amount	Content	Amount	Content
Lump-sum payments	80	Asahi Kasei Pharma, Lundbeck	209	RQ-10, Motilin, 5-HT2B, etc	300	RQ-4	363	RQ-10, Motilin (collaborative research part), 5-HT2B
Milestone payments	20	CJ Healthcare	20	CJ Healthcare	250	Aratana Inc. (RQ-5)	775	Aratana Inc. (RQ-7)
Royalty income	0		0		0		63	Aratana Inc (animal drug)
Joint research collaboration income	128	Ajinomoto Pharmaceutical, Eli Lilly	71	Ajinomoto Pharmaceutical, Asahi Kasei Pharma, others	50	Asahi Kasei Pharma, others	0	
Total	228		300		600		1,200	

Source: prepared by FISCO from company materials

The above table focuses on the performance forecasts in the medium-term management plan, but in FY12/17 after the plan, Aratana's animal drug would be well accepted in the market, (ghrelin receptor agonist, RQ-5) and in addition, the same company's animal drug for osteoarthritis (EP4 antagonist, RQ-7) should have been launched, and the royalty income from these drugs is expected to constitute the core of the company's earnings. Also, on entering FY12/18, in addition to these two animal drugs, in the human drugs area it is possible that CJ Healthcare will have marked launched the acid pump antagonist (RQ-4), that Dalbavancin will have been launched in the Japanese market, and that Meiji Seika Pharma will have launched Ziprasidone. If the company reaches this stage, then judging from factors such as the expected market scales, it can reasonably expect to receive royalty income in excess of ¥2 billion, which should enable it to stably achieve profitability.

It is reducing business expenses by transferring its R&D base

(3) Details of expenses forecasts

With regards to business expenses also, the company is maintaining a sufficient research and development system and plans to reduce its expenses during the period of the current medium-term management plan. It required business expenses of ¥2.366 billion in FY12/13, but it plans to reduce this by 29% to ¥1.686 billion in FY12/16.

Breaking down its business expenses by item, it is mainly aiming to reduce facilities-related expenses and it plans to reduce this item by ¥535mn by FY12/16 compared to the total in FY12/13. A major factor behind this will be that the company has established an industry-academic joint research division in Nagoya University and is transferring its research and development base to it. In other words, it should be able to reduce its facilities expenses by transferring its research and development base from the research facility it rented from Pfizer to within the campus of Nagoya University.

The company has been steadily transferring its research and development functions to this new location since April 2014, and it has also transferred its head office functions to an easily accessible location in front of Nagoya Station. Its other cost items in its FY12/14 interim financial statements are roughly in-line with forecasts.

Break-down of results and forecasts for business expenses by item

(unit: ¥mn)

	FY2013 Result	FY2014 Plan	FY2015 Target	FY2016 Target
Business expenses, total	2,366	1,984	1,698	1,686
Personnel expenses	859	708	682	682
R&D expenses	429	557	481	457
Administrative expenses	299	289	287	286
Facilities expenses	641	307	124	106
Others	135	119	121	154

Source: prepared by FISCO from company materials

It is set to reach its fund raising target of ¥2 billion yen for FY12/14

(4) Required capital and fund raising

The biggest problem not just for the company but for every drug discovery venture company is how to secure the capital it needs during the period before it is able to stably acquire royalty income.

In order to secure the funds it needs during this period and when assuming the above mentioned business earnings and expenses, the company's basic policy is to raise funds of around ¥2 billion yen a year. If it can achieve this, it would secure a cash balance of around ¥4 billion at the end of each period, which is considered to be sufficient for it to conduct its research and development activities without any funding concerns. In terms of its specific methods of raising funds, the company employs a variety of methods, including the exercising of share options (capital increase through an allotment to a third party), implementing project financing, and utilizing its asset holdings.

During FY12/14, in February 2014 the company raised funds by selling the investment securities it held in Aratana of the United States for approximately ¥1.8 billion (¥1.5 billion yen capital gain). Also, in July 2014, it issued 10th stock options through an allotment to Merrill Lynch Japan Securities. If the exercise of these options goes as planned, it will raise around ¥1.366 billion. In FY12/14, it has practically achieved its target of raising around ¥2 billion, but going forward it will be necessary to pay attention to this point to confirm that it can continue its trials in subsequent periods.

It has basically achieved its forecasts in its 1H financial statements

(5) Financial statements in 1H FY12/14 (January to June)

In its financial statements in 1H FY12/14, the company recorded sales of ¥95mn (an increase of 16.0% year on year (YOY)), an operating loss of ¥993mn (compared to an operating loss of ¥1,133mn in the same period in the previous fiscal year), a recurring loss of ¥1,060mn (a recurring loss of ¥874mn), and net income of ¥461mn (a net loss of ¥903mn). The company did not publish forecasts in its 1H financial statements and therefore we cannot provide an analysis compared to forecasts, but its performance was more or less in-line with other forecasts. Its forecasts for the FY12/14 full year are unchanged from those in its medium-term management plan.

The details of the progress the company has made for its research and development activities are as previously described for each program.

Income statement

(unit: ¥mn)

	FY12/10	FY12/11	FY12/12	FY12/13			FY12/14		
				1H	2H	Full year	1H	2H (E)	Full year (E)
Sales	1,186	684	28	82	146	228	95	205	300
R&D expenses	1,652	1,660	1,804	791	727	1,518	663	-	-
SG&A expenses	777	928	861	424	423	847	422	-	-
Operating income	(1,345)	(1,916)	(2,636)	(1,133)	(1,004)	(2,137)	(993)	(691)	(1,684)
Ordinary income	(1,295)	(1,906)	(2,891)	(874)	(945)	(1,819)	(1,060)	(625)	(1,685)
Net income	(1,307)	(1,916)	(2,905)	(903)	(205)	(1,108)	461	(743)	(282)
Income per share	(652.74)	(172.85)	(219.00)			(82.70)			(20.82)
Dividend per share	0.00	0.00	0.00			0.00			0.00
Net assets per share	452.26	616.14	400.27			423.84			
Equity ratio	94.0	97.6	96.5			85.9	84.0		

Source: prepared by FISCO from company materials

FISCO

RaQualia Pharma Inc.
4579 JASDAQ

5-Sept.-14

Balance sheet

(unit: ¥mn)

	FY12/10	FY12/11	FY12/12	FY12/13	1H FY12/14
Current assets	3,845	7,783	5,089	4,363	5,145
Cash and deposits	3,392	7,672	4,889	4,035	3,267
Accounts receivable-trade	353	1	9	59	7
Inventory	50	45	47	46	33
Other	48	64	142	221	1,837
Noncurrent assets	615	595	411	2,284	805
Property, plant and equipment	70	68	101	7	41
Intangible assets	26	26	20	11	13
Investments and other assets	518	501	288	2,265	751
Assets, total	4,460	8,379	5,501	6,648	5,950
Current liabilities	269	204	183	232	869
Notes and accounts payable	0	0	0	0	0
Short term debt	0	0	0	0	110
Accounts payable-other	193	99	90	141	645
Other	76	105	92	91	114
Noncurrent liabilities	0	0	7	669	47
Long term debt	0	0	0	0	0
Other	0	0	7	669	47
Shareholders' equity	4,199	8,203	5,298	4,466	4,927
Capital stock	5,529	8,489	8,489	8,627	8,627
Capital surplus	813	3,773	3,773	3,911	3,911
Retained earnings	(2,143)	(4,060)	(6,965)	(8,073)	(7,611)
Treasury stock	0	0	0	0	0
Other	(8)	(29)	12	1,246	70
Subscription rights to shares	0	0	0	33	34
Total net assets	4,191	8,174	5,310	5,746	5,033
Total liabilities and net asset	4,460	8,379	5,501	6,648	5,950

Source: prepared by FISCO from company materials

Statement of cash flow

(unit: ¥mn)

	FY12/10	FY12/11	FY12/12	FY12/13	1H FY12/14
Net cash provided by (used in) operating activities	(1,470)	(1,590)	(2,728)	(2,179)	(1,097)
Net cash provided by (used in) investing activities	(465)	(3,810)	3,741	951	243
Net cash provided by (used in) financing activities	1,622	5,897	0	309	110
Effect of exchange rate change on cash and cash equivalents	(4)	(11)	4	63	(31)
Net increase (decrease) in cash and cash equivalents	(318)	484	1,012	(854)	(774)
Cash and cash equivalents at beginning of period	3,710	3,392	3,877	4,889	4,035
Cash and cash equivalents at end of period	3,392	3,877	4,889	4,035	3,260

Source: prepared by FISCO from company materials

■ Returns to shareholders

It may start paying dividends comparatively soon after its performance stabilizes

The company is at a stage where it is still posting a net loss, so it does not provide dividends to shareholders. However, its awareness of returning profits to shareholders is extremely high. It is a company that is very reliant on its staff, such as its researchers, and also a company that is involved in drug development that carries with it a significant social responsibility, and therefore the company management is highly aware that it must contribute and return to a wide range of stakeholders, not just to its shareholders.

It is thought that such as management stance improves the motivation of its talented staff, which results in the development of better products, which in turn is reflected in improved performance. In the future, judging from the awareness that the company management has about the importance of contributing and returning to stakeholders, it is likely that comparatively soon after it stabilizes its performance it will start paying dividends to shareholders.

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