RaQualia
innovators for life

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Medium-Term Management Plan Gaia 2021 (2020 - 2022)

Released on Feb. 14, 2020　(TSE JASDAQ Growth: 4579)

RaQualia Pharma Inc.

Topics

tegoprazan South Korea Launched in Mar. 2019; China Phase 3 in progress

GALLIPRANT® Sustained strong sales performance in U.S.; Europe launched in Mar. 2019

ENTYCE® Steady sales performance in U.S.; Pivotal trials in cats in progress

Expansion of alliance: Expansion of global partnership with CJ HealthCare Corporation ("CJ") agreed;
tegoprazan licensed to CJ in North America and Europe

Out-licensed programs:

P2X7 receptor antagonist --- Stage-up at Asahi Kasei Pharma Co., Ltd.

Specific ion channel drug --- Stage-up at EA Pharma Co., Ltd.

Ziprasidone --- Meiji Seika Pharma Co., Ltd.reported topline results of Phase 3 trials in Japan

Joint research projects(new):

ASKA Pharma Co.,Ltd.(ion channel)

Epigeneron Co., Ltd.(kidney disease)

Nagoya City University (muscular atrophy lateral sclerosis / infectious disease)

Gifu Pharmaceutical University (retinal venous occlusion)

Academic conference presentation:

Joint poster presentation on corticotropin releasing hormone receptor 2 (CRHR2) modulator with Nagoya University

Consolidated Statements of Income for FY2019

(Unit: Millions of yen)

	FY2018 (Previous term)	FY2019 (Current term)
Operating revenues	744	**1,702**
Operating expenses	1,819	**1,718**
Operating profit (loss)	(1,075)	**(15)**
Ordinary profit (loss)	(1,064)	**21**
Profit attributable to owners of the parent (loss)	(1,104)	**5**

Curbed from 1,840 (as of Sep.6 forecast)

First profit since RaQualia was established

Forecast for FY2020

(Unit: Millions of yen)

[Consolidated]	FY2019 (Results)	FY2020 (Forecast)
Operating revenues	1,702	2,129
Operating expenses and operating costs	1,718	2,059
Operating income (loss)	(15)	70
Ordinary income (loss)	21	85
Profit attributable to owners of the parent (loss)	5	13

Topics

- Operating revenues: We expect royalty income to increase further due to steady sales of tegoprazan (Korean brand name: "K-CAB® ") in South Korea and solid sales of two pet-use drugs, GALLIPRANT® and ENTYCE®.
- We will promote licensing and alliance activities and expect to receive upfront payments and milestone payments.
- Operating expenses and operating cost: We will make investments to advance each project at the exploratory stage and early development stage, while continuing to optimize operating expenses and other expenses in an effort to strengthen our earnings structure.

Major Events in FY2020 (1)

Strong sales of tegoprazan in South Korea; global developments progress.

- Full-year contribution of tegoprazan (South Korean product name: K-CAB®) in FY2020 (CJ launched in Mar. 2019).
- In Nov. 2019 we entered into a global partnership agreement with CJ. CJ took over the phase I clinical trials we conducted in the U.S. We plan to resume development in the U.S. during the term ending Dec. 2020.
- In Japan, we are strengthening our cooperative relationship with CJ.
- Phase III clinical trials of tegoprazan are proceeding smoothly by Luoxin in the China.

Sales of pet medicines remained solid.

- Sales of GALLIPRANT® (grapiprant/RQ-00000007) in the U.S. and Europe are growing steadily. The sales territory may expand.
- Steady progress in U.S. sales of ENTYCE® (capromorelin/RQ-00000005), a treatment for anorexia in dogs. Pivotal trials for older cats with chronic kidney disease are proceeding smoothly in the U.S.

Syros Pharmaceuticals Inc. (U.S.) is steadily developing Tamibarotene.

- Steady progress in Phase II clinical trials in combination with 5-azacitidine (Bristol-Myers Squibb) in naive and relapsed/refractory AML patients.

Major Events FY2020 (2)

Further strengthening our core competencies

- Further progress in ion channel drug discovery. Seeking new joint research with pharmaceutical companies.
- Continuous strengthening of intellectual property. Promotion of a life cycle management (LCM) strategy.

Expectations for clinical development in alliance partners

- Clinical development of the P2X7 receptor antagonist (RQ-00466479/AKP-23494954) steadily proceeded at Asahi Kasei Pharma. Further progress is expected.
- Clinical development of compounds targeting ion channels created through collaborative research with EA Pharma (compound codes not disclosed) steadily proceeded at EA Pharma.

Other

- Our U.S. branch (US BRANCH) is fully operational. The branch aims to build good relationships with local academia, medical institutions, and venture companies.
- RaQualia Innovations Inc. promotes information-gathering activities to contribute to the discovery of new alliance candidates and the cultivation of new therapeutic areas and modalities, aiming to generate synergies with RaQualia's core technologies. The company transitioned into new deals by building stronger relationships with potential clients.

CEO Message

Twelve years have passed since our founding as a drug discovery venture. **We are now shifting into a Stabilization Phase with Royalty Revenue.**

We aim to make further progress as an **R&D-driven Company** in the future.

Naoki Tani, Representative Director,
RaQualia Pharma Inc.

Outline of the “Gaia 2021" Medium-Term Management Plan

Basic Policies of the Medium-Term Management Plan

- **Realizing our vision**

  “We seek to bring people greater health and happiness through innovative new medicines."

- **Presenting and implementing specific measures to stabilize management and increase shareholder value**

- **Aiming to evolve on a "global" scale with creative power like the "Gaia" earthworm**

Outline of the Medium-Term Management Plan

➢ **Building and strengthening a drug discovery research platform aimed at early out-licensing**

- Continue and strengthen innovative drug discovery joint research centered on industry-academia-government collaboration.
- Continuously create new development compounds through our in-house evaluation system.

➢ **Launch of human-use pharmaceuticals and establishment of an efficient out-licensing system**

- Establish and strengthen an efficient licensing-out system for early out-licensing of existing pipelines.
- Build relationships of trust and secure royalty income through cooperation and collaboration with partner companies.

➢ **Acceleration of overseas operations**

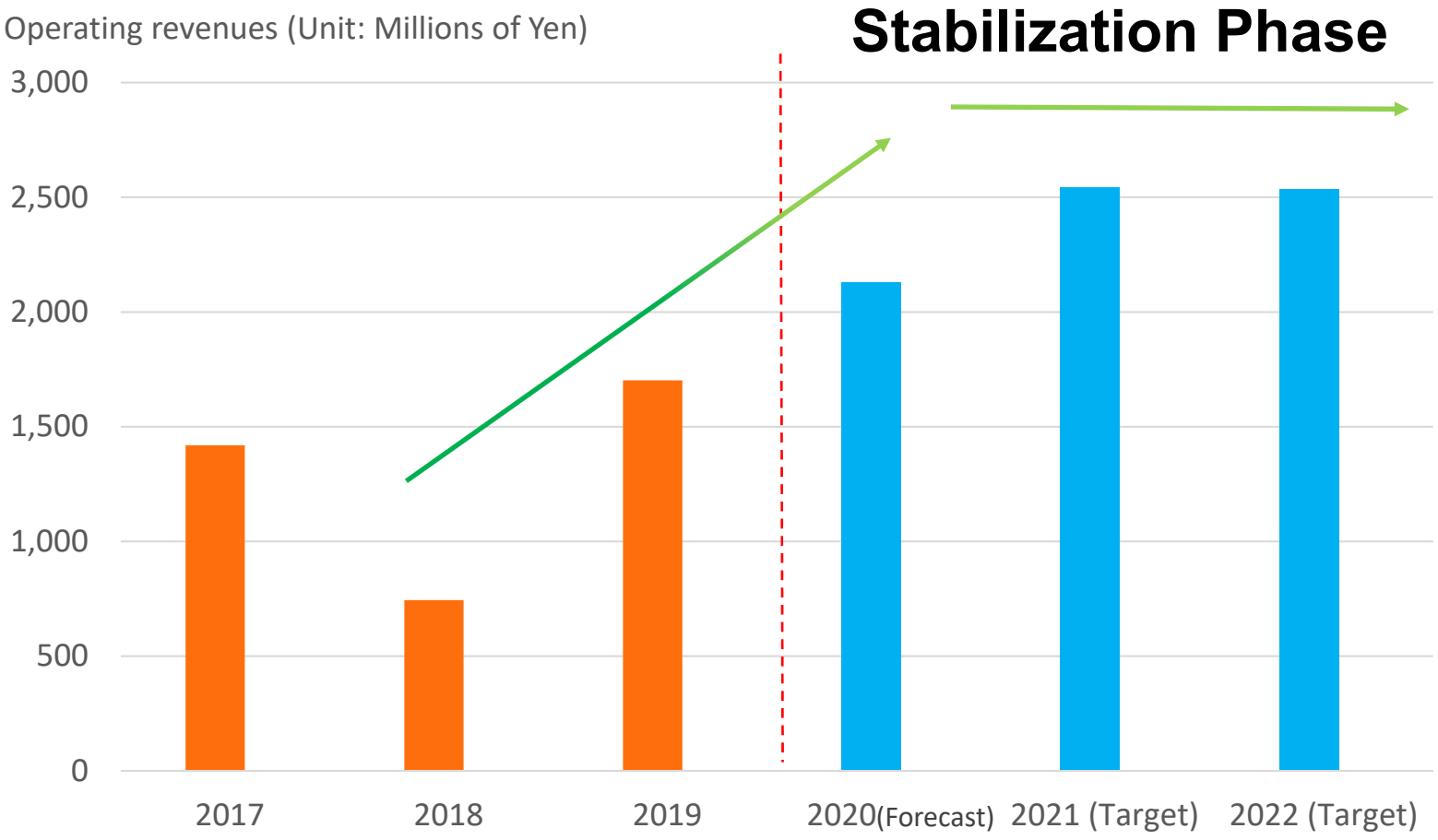
- Set up a U.S. branch in San Diego, California, a city with an established innovation ecosystem for pharmaceutical companies and other entities; begin full-scale information-gathering activities.

Medium-Term Management Plan (Gaia 2021) overview

(Unit: Millions of yen)

[Consolidated]	FY2018 (Results)	FY2019 (Plan as of Sep. 6)	FY2019 (Results)	FY2020 (Forecast)	FY2021 (Target)	FY2022 (Target)
Operating revenues	744	1,756	1,702	2,129	2,543	2,535
Operating expenses	1,819	1,840	1,718	2,059	2,209	2,168
(Labor costs)	607	625	616	705	770	789
(R&D expenses)	451	267	221	396	358	360
Operating income (loss)	(1,075)	(84)	(15)	70	334	367
Ordinary income (loss)	(1,064)	(82)	21	85	362	384
Profit (loss) attributable to owners of the parent company	(1,104)	(106)	5	13	247	322
Foreign exchange rates (U.S. dollars and Japanese yen)	110.91	110.00	109.55	110.00	110.00	110.00

Changes in business earnings



Steady increase in operating revenues from up-front and milestone payments.
Shift to a stabilization phase with royalty revenues.

Overview of expenses

(Unit: Millions of yen)

	FY2018 (Results)	FY2019 (Plan as of Sep.6)	FY2019 (Results)	FY2020 (Forecast)	FY2021 (Target)	FY2022 (Target)
① Operating costs	89	272	262	224	348	370
② Operating expenses	1,730	1,568	1,456	1,835	1,861	1,798
Labor costs	607	625	616	705	770	789
R&D expenses	451	267	221	396	358	360
Administrative and Control expenses	255	273	236	317	288	288
Facility-related expenses	204	235	221	223	255	206
Others	213	168	162	194	190	155
Total (①+②)	1,819	1,840	1,718	2,059	2,209	2,168

FY2020

Labor costs increase with planned recruitment of researchers in order to expand the drug discovery base.
R&D expenses increase in step with higher commissioned research expenditure to promote the development of in-house programs.
General expenses increased in step with accelerated R&D activities and increased support from specialists.

FY2021

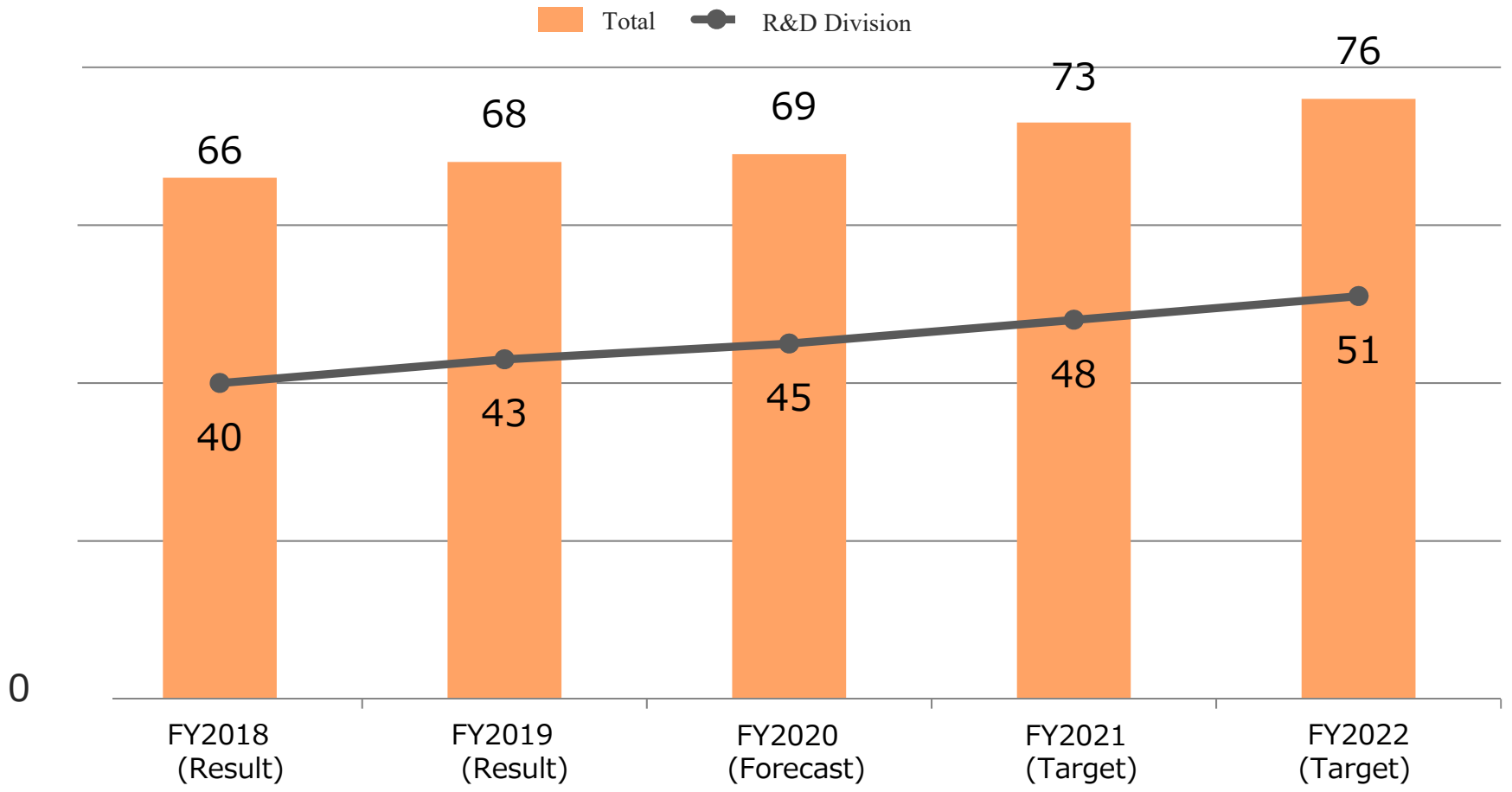
Labor costs increase with planned recruitment of researchers.
R&D expenses decrease with the completion of outsourcing R&D expenses.

FY2022

Labor costs increase with planned recruitment of researchers.

Personnel plan

As of the date of submission



※. Including persons on a consolidated basis and persons on leave

※. Excluding temporary staff

Fundraising strategy

Basic Policy

- Maintain fund balance at the level of ¥3.0 billion at each fiscal year end by continuing cost reduction measures implemented the year before last.
- Running costs should be basically covered by business revenue and cost reduction measures.
- Devote budget surplus to Discovery research activities with the aim of enhancing the value of existing programs and monetizing the achievements.
- Propose and implement financing strategies with a convincing equity story based on shareholder value.

Results (cash and deposits, investment securities, etc.)

- Balance of funds at the end of FY2019 (approx. ¥3.6 billion)

Funding option

- Allocation of new stocks to a third party for stable investors
- Public stock offerings
- Consider a new way of financing (e.g. financing per project)
- Financing with effective use of assets held
- Debt

Rooted in shareholder value improvements that the market can understand. Presentation and implementation of funding strategies with a clear equity story.

Review of FY2019 (Business Highlights)

1) Tegoprazan (Slides 15-18)

2) Pet Phamaceuticals (Slides 19-20)

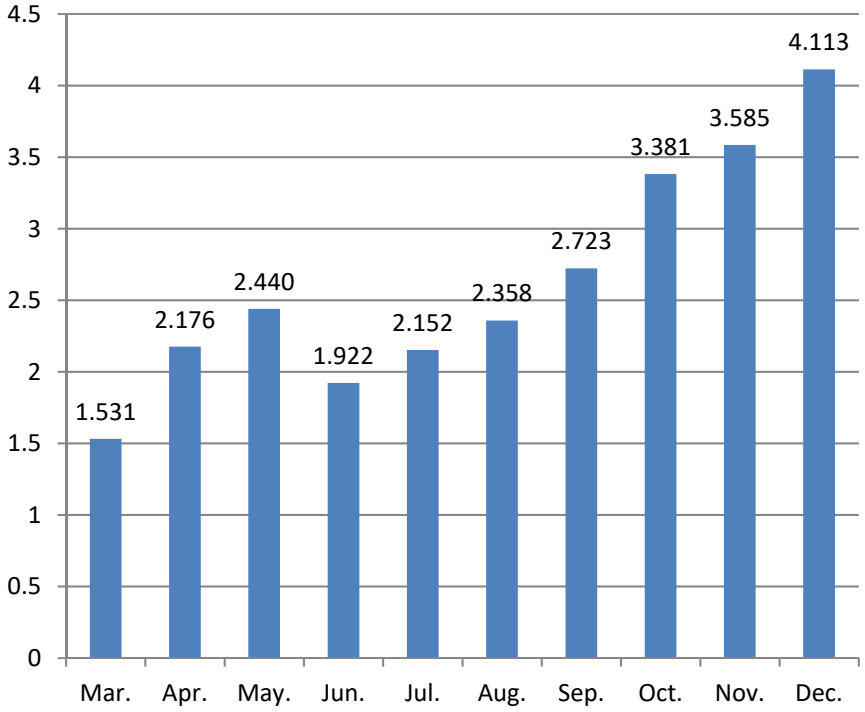
3) Ion Channel Drug Discovery (Slide 21)

4) TMRC; Tamibarotene (Slides 22-24)

1) tegoprazan: K-CAB ® makes a good start in South Korea

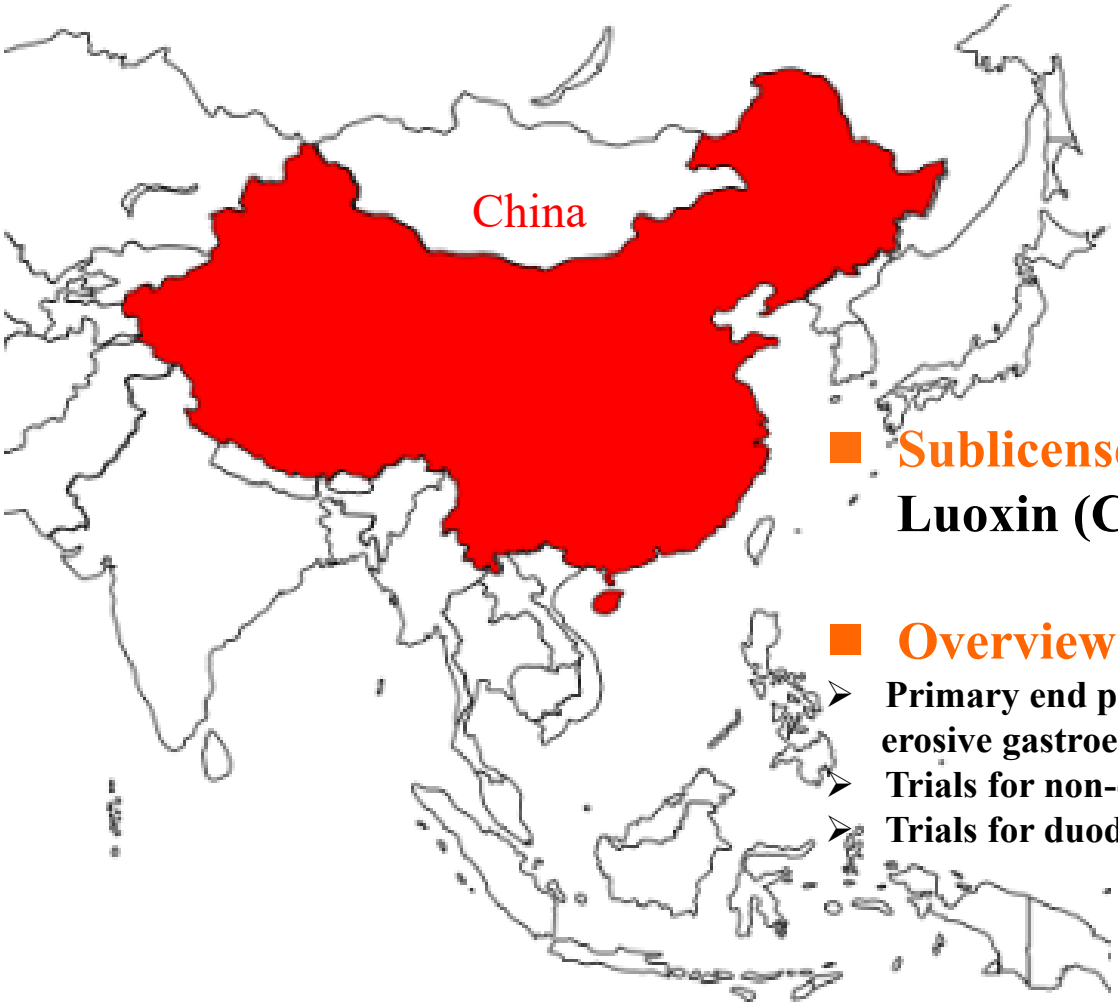
Marketing pamphlet

Sales in South Korea
from Mar.through Dec.,2019



Source: UBIST
Outpatient prescription data (sales, bw)

1) tegoprazan: Chinese phase 3 trials on going



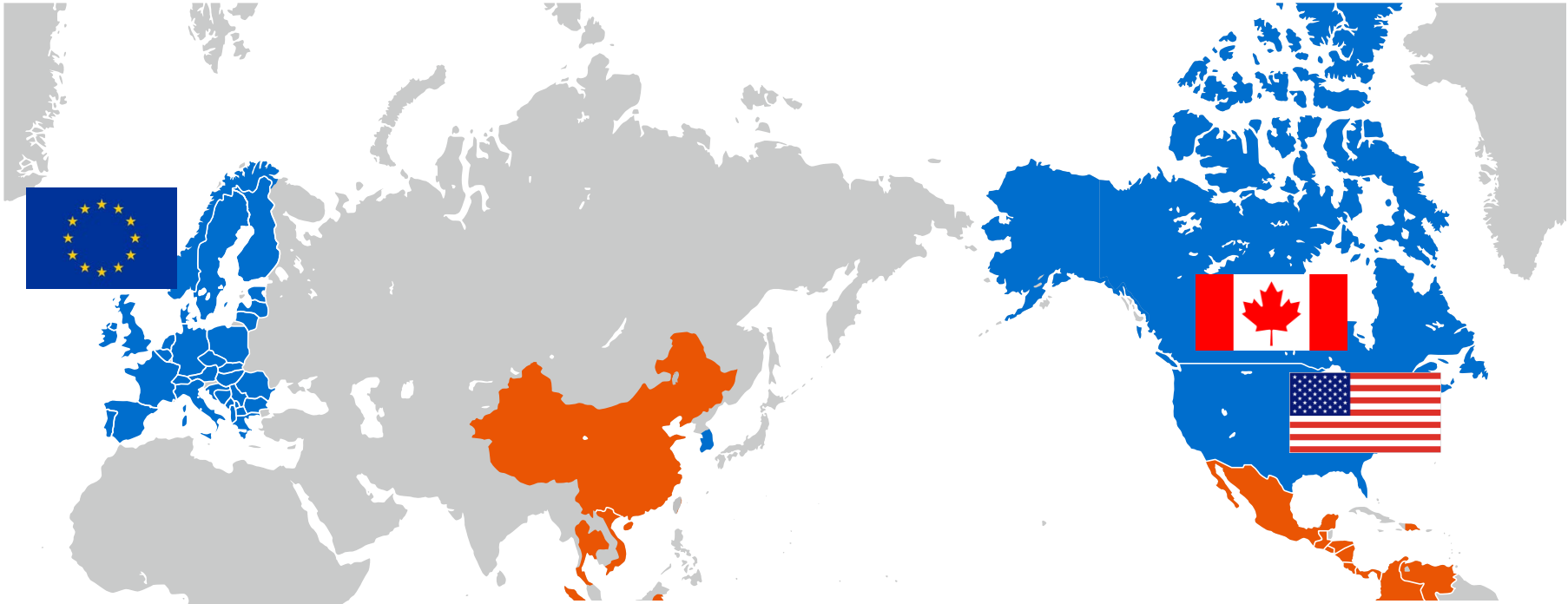
Sublicensee

Luoxin (China)

Overview of Phase III Clinical Studies

- **Primary end point achieved for targeted indication of erosive gastroesophageal reflux**
- **Trials for non-erosive gastroesophageal reflux in progress**
- **Trials for duodenal ulcer under consideration**

1) tegoprazan: Expansion of global partnership



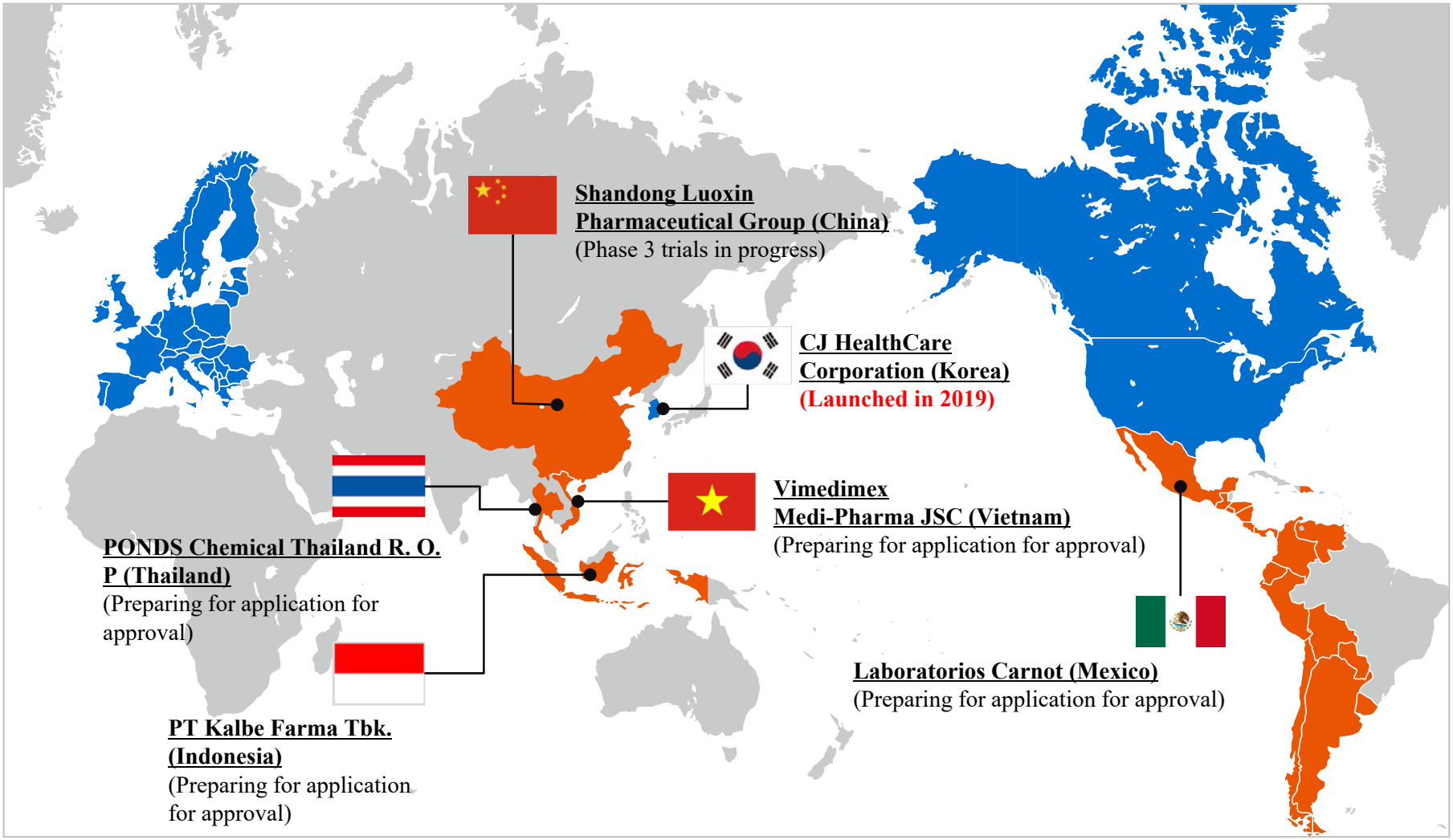
Nov. 26, 2019

Expansion of global partnership with CJ
North America and Europe: Grant exclusive tegoprazan licenses

Entry into the bigger markets of the U.S. and Europe.

Stronger alliance with CJ

1) tegoprazan: Global Expansion (States of sublicensees)



2) Pet pharmaceuticals: GALLIPRANT®

Galliprant® (grapiprant tablets)

Indication	Dog Osteoarthritis (chronic inflamatory pain)
Distributor	Elanco Animal Health Inc. (“Elanco") (July 2019: Elanco acquired Aratana Therapeutics Inc. as a subsidiary; new marketing structure being built
Reference Information	**Leading brand in nonsteroidal anti-inflammatory analgesic (NSAID)**

2) Pet pharmaceuticals: ENTYCE®

entyce
(capromorelin oral solution)

Indication	Appetite Stimulation (weight loss)
Distributor	Elanco
Reference Information	・Enhanced marketing promotion by Elanco ・Pivotal study in older cats with chronic kidney disease in the U.S. ongoing

3) Ion channel drug discovery

Joint research conducted with four pharmaceutical companies

Company Name	Contents	Recent Situation
EA Pharma	Joint research targeting specific ion channels in the gastrointestinal field	Sep. 2019 Milestone achievement
Maruho	Selective sodium channel blockers	currently under development
Asahi Kasei Pharma	P2X7 receptor antagonist (RQ-00466479, AKP-23494954) Target indication: Neuropathic Pain	Oct. 2019 Milestone achievement
ASKA Pharmaceutical	Collaborative research targeting specific ion channels	Jul. 2019 Collaborative research agreement signed

- Joint research in the exploratory research stage

Moving forward from the exploratory research stage, we will leverage the combined strengths of the Company and pharmaceutical company collaborators to create innovative development compounds

4) TMRC Co., Ltd.: Development of Tamibarotene

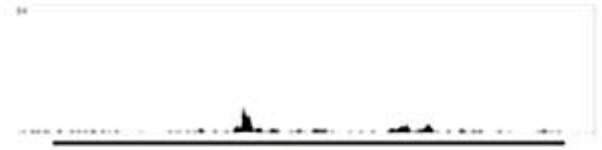
- A Phase 2 study on acute myeloid leukemias (AML) is underway to assess the efficacy and safety in combination with 5-azacitidine (Bristol-Myers Squibb) in patients who have not received any treatment or who have recurrned or refractory to other treatments. The interim results are favorable.
- Breast cancer (BC) :Begin a trial in light of the effectiveness of the AML combination trials.
- Neuroblastoma (NB): Establish a POC in combination with an epigenetic drug are acceralate further development toward a regulatory marketing approval.
- Acute promyelocytic leukemia (APL) in China: Seek on import approval in China for ATRA recurrences and refractory APL in combination with arsenite.
- Neutropenia (NP): The Company aims to enter into a contract as early as possible in the process of out-licensing negotiations, and to expand into areas other than cancer indications.

Indication	Out-licensing partner	Search / Pre-clinical / Clinical trials (Phase 1 / Phase 2 / Phase 3) / Appli-cation / Approval / Marketing	Contract Region
Acute Myeloid Leukemia: AML	Syros Pharmaceutical, Inc. (U.S.)	Phase 2 in progress	United States
Breast Cancer: BC	Syros Pharmaceutical, Inc. (U.S.)	Preparing for Phase 2	United States
Neuroblastoma: NB	OHARA Pharmaceutical Co.,	Phase 1/2 in progress	Japan
Acute Promyelocytic Leukemia: APL	TOKO Pharmaceutical CO.,	In progress	China
Neutropenia: NP	Out-licensiy negotiations in progress	Completed all pre-clinical testing	Asia

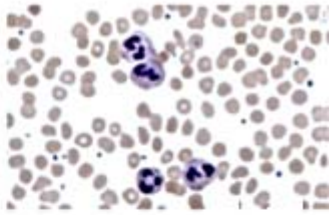
Note: This document is not prepared for the purpose of soliciting investment. Investors are advised to make their own judgments when making investments. Please refer to the notes at the end of the final page of this document.

4) TMRC Co., Ltd.: Mechanism of Tamibarotene

① Normal myeloid cells

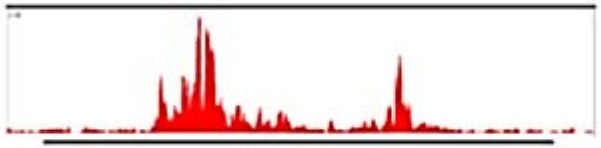


Retinoic acid α gene

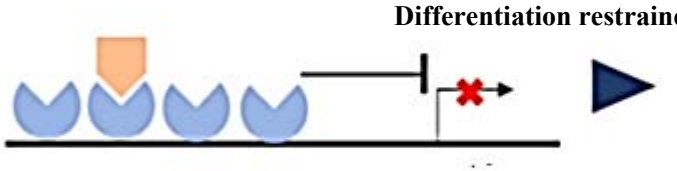


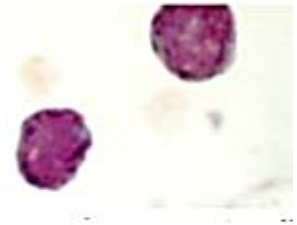
Normal myeloid formation

② Acute Myeloid Leukemia (myeloid cancer)



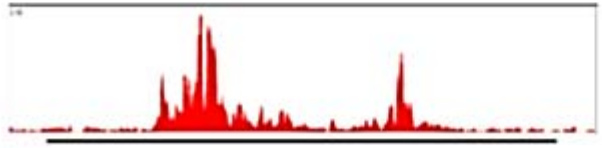
Retinoic Acid α Gene Over-Expression





Cancer/undifferentiated

③ Acute Myeloid Leukemia + Tamibarotene

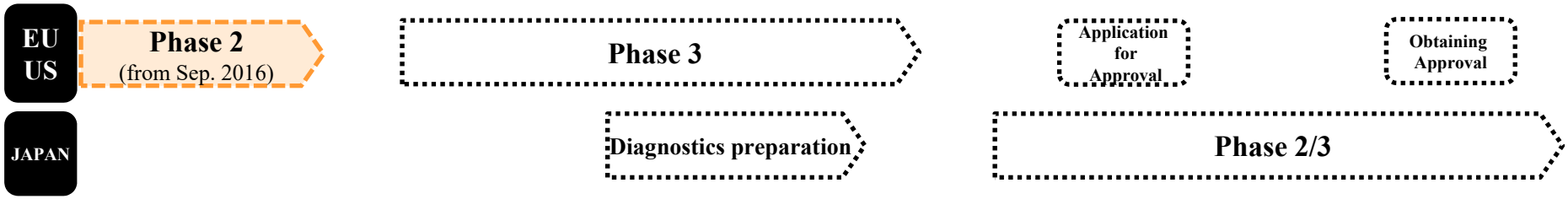


Retinoic Acid α Gene Over-Expression

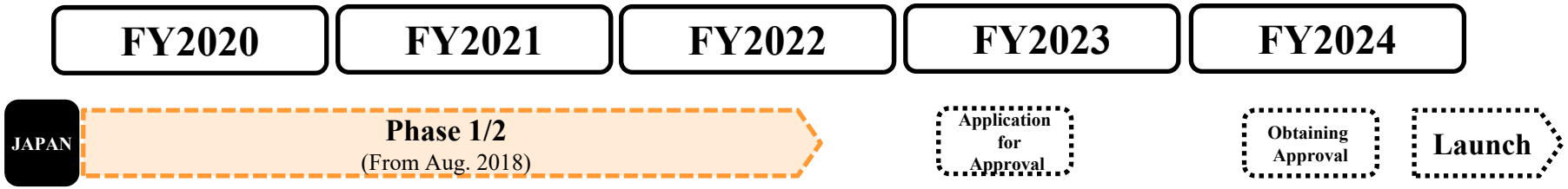
Normal myeloid formation

4) TMRC Co., Ltd.: Tamibarotene Roadmap

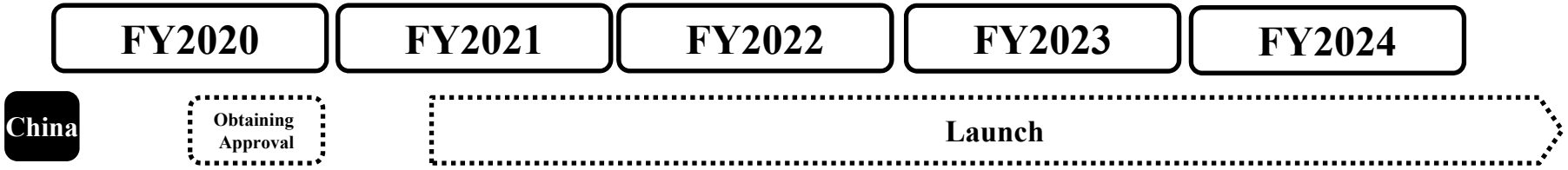
Acute myeloid leukemia (AML)



Neuroblastoma (NB)



Acute promyelocytic leukemia (APL)



Scheduled: 3rd Quarter

Major out-licensed programs (for human beings)

Project	Compound (Generic name)	Out-licensing partner	Primary indication	Search	Preclinical	Clinical trials			Appli-cation	Approval	Marketing
						Phase 1	Phase 2	Phase 3			
Ziprasidone	RQ-00000003	Meiji Seika Pharma	Schizophrenia					●			Phase 3 in progress (Japan)
Potassium-ion competitor Acid Blocker (P-CAB)	RQ-00000004 (tegoprazan)	CJ Healthcare	Gastroesophageal reflux disease					●	○	○	● Launched in 2019 (Korea)
								●			Phase 3 in progress (China)
						●					Phase 1 completed (U.S.)
EP4 antagonists	RQ-00000007 (grapiprant) RQ-00000008	AskAt	Cancer			●					Phase 1 in progress (U.S.)
						●					Phase 1 in progress (China)
			Pain				●				Phase 2 a completely in the previous fiscal year (U.S.)
						●					Phase 1 in progress (China)
COX-2 inhibitors	RQ-00317076	AskAt	Pain				●				Phase 2 a completely in the previous fiscal year (U.S.)
						●					Phase 1 in progress (China)

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Derivation Preparation Program

(As of the date of submission)

- Potassium-ion-competitor acid blocker:Completed Clinical study report on P-CAB (tegoprazan/RQ-00000004) for Phase 1 (Dec. 2016)
- Completed Clinical study report on 5-HT_4 partial agonist (RQ-00000010) completed for Phase 1(Oct. 2017)
- Completed Clinical study report on 5-HT_{2B} antagonist (RQ-00310941) for Phase 1 (Apr. 2018)
- Completed Pre-clinical trials on motilin-agonist (RQ-00201894) ; Phase 1 clinical trials are under consideration
- Pre-clinical trials are under consideration for the ghrelin-agonist (RQ-00433412) and TRPM8 blocker (RQ-00434739)

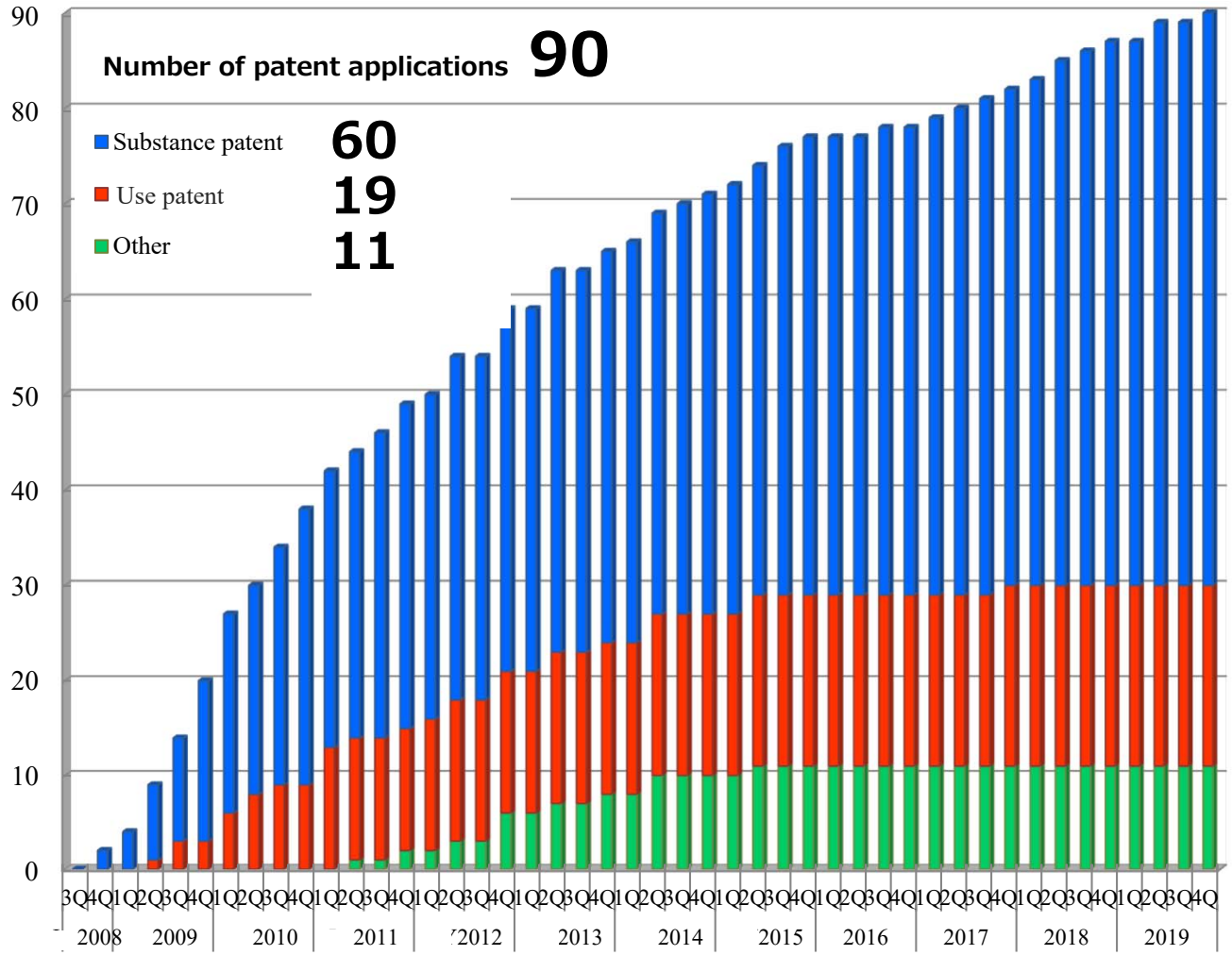
Project	Compound	Primary indication	Search	Pre-clinical	Clinical trials			Appli-cation	Approval	Marketing	Implementation Region
					Phase 1	Phase 2	Phase 3				
Potassium-ion competitor acid blocker (P-CAB)	RQ-00000004 (tegoprazan)	Gastroesophageal reflux disease (GERD)			Completed of Phase 1						Japan
5-HT_4 partial agonist	RQ-00000010	Gastric paresis Functional dyspepsia Chronic constipation			Completed of Phase 1						United Kingdom
5-HT_{2B} antagonist	RQ-00310941	Diarrhea-type irritable bowel syndrome (IBS-D)			Completed of Phase 1						United Kingdom
Motilin agonist	RQ-00201894	Gastroparesis Functional dyspepsia Post-operatiue ileus			Under consideration						Japan
Ghrelin receptor agonist	RQ-00433412	Cancer-related Anorexia/Cachexia syndrome		Under consideration							Japan
TRPM8-Blocker	RQ-00434739	Neuropathic pain (cold allodynia induced by chemotherapy)		Under consideration							Japan

Patent news

Date	Target	Region	Contents
Dec. 20, 2019	Selective sodium channel blockers (Amide derivatives)	Korea	Substance patent
Oct. 29, 2019	Selective TRPM8 blockers (Azaspiro derivative)	Japan	Substance patent
Aug. 6, 2019	New applications for potassium-ion competitive acid blockers (P-CAB)	Europe	Use patent
Jul. 23, 2019	Nav1. 7 and Nav1. 8 sodium channel blockers (Amide derivatives)	Japan	Substance patent
Apr. 9, 2019	Ghrelin receptor agonists (Serine derivatives)	Korea	Substance patent

Patent Applications

(excluding transactions transferred to each country)



Industry-Academia-Government Collaboration

Search for selective inhibitors of specific enzymes for the development of drugs for refractory neuro blastoma (May. 2015)

Kenji Kadomatsu, Professor of Biochemistry, Nagoya University Graduate School of Medicine

Exploration of selective inhibitors for specific proteins for the development of heart failure drugs (Oct. 2015)

Mitsuhito Takefuji, Assistant Professor of Cardiology Science, Graduate School of Medicine, Nagoya University

Search for a drug to treat nonalcoholic steatohepatitis (NASH) (started in Sep. 2016; renewed in Oct. 2018)

Takayoshi Suganami, Professor of Research Institute of Environmental Medicine Nagoya University (in the field of Molecular Metabolism)

Development of a New Drug for the Treatment of Mutant KRAS Lung Cancer Using Cellular Aging (Jan. 2018)

Mitsuo Sato, Professor of Nagoya University Graduate School of Medicine

Elucidation of the Mechanism of TRPM8 Blocker (RQ-00434739) at the Core (Oct. 2018)

Makoto Sawada, Professor of Reseach Institute of Environmental Medicine Nagoya University (in the field of Brain Function)

Exploration of drugs for the treatment of retinal vein occlusion (RVO) (May. 2019)

Hideaki Hara, Professor of Molecular Pharmacology Gifu Pharmaceutical University

Initial exploratory research aimed at the creation of new therapeutic agents for ALS (ALS) (Jul. 2019)

Mr. Hitomi Tsukiji, Hospital Eco-Chemical Sector, Graduate School of Pharmacy, Nagoya City University

Development of a new treatment for macrolide-resistant group A streptococci (Dec. 2019)

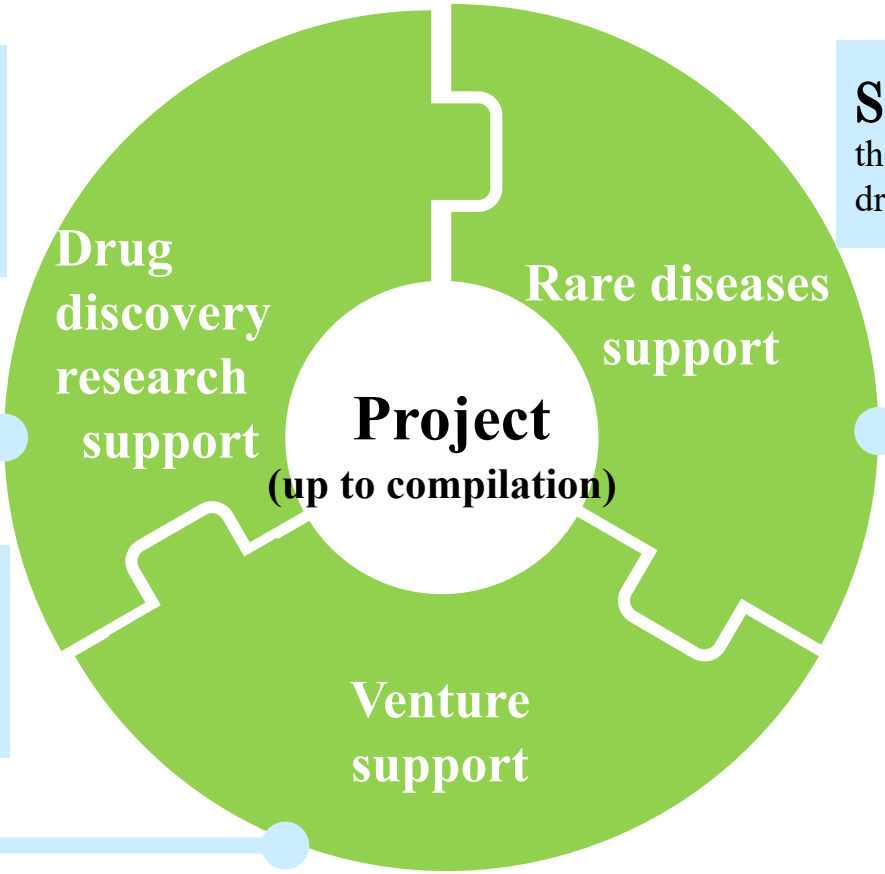
Professor Tadao Hasegawa, Department of Bacteriology, Department of Medicine, Nagoya City University

RaQualia Innovations K.K

Promote the commercialization of drug candidate compounds originating from academia

Provide optimal solutions and promoto collaboration for biotech ventures

Support the development of orphan drugs for children



◆ To assist ventures and academia in the life sciences for the development of technologies and the formulation of intellectual property strategies, and for the building of relationships and structuring of deals

◆ In FY2020: Strengthen collaboration with venture companies identify as candidates for support, and aim for project origination

Notes on Business Projections

- Materials and information provided in this document include forward-looking statements. These statements are based on the current expectations, projections and assumptions involving risks, and contain uncertainties that could cause the actual results to be substantially different from such statements.

- Such risks and uncertainties include general conditions of domestic and global economy such as general industry and market conditions, interest rate, changes in exchange rates. Particularly, risks and uncertainties exist in forward-looking statements related to products (R&D programs and compounds). Risks and uncertainties of products include, but not limited to, technical progress, acquisition of patents by competitors, completion of clinical trials, claims and concerns regarding safety and efficacy of products, approval by regulatory authority, health-care insurance reforms within and outside Japan, tendency to contain medical expenses, laws and regulations of the government affecting businesses within and outside Japan, challenges associated with new product development.

- The Company does not undertake to update or revise any forward-looking statements contained in this document, whether as a result of new information, future events, or otherwise.

Note: This document has not been prepared for the solicitation of investments. In making any investments, investors are kindly required to base their decisions on their own judgments. Please be sure to read the notes at the end of this paper, without fail.

RaQualia
innovators for life

Contact Information

E-mail: Our website, please.
https://www.raqualia.co.jp/(Link)

RaQualia Pharma Inc.

appendix

Company overview

As of the date of submission

Company name

RaQualia Pharma Inc.

Representative Director

Naoki Tani

Description of Businesses

Pharmaceutical products and clinical development candidates in pharmaceutical R&D; sales and licensing of intellectual properties of the related core technologies

Employees

69 persons

Establishment

Feb. 19, 2008

Common stock

¥2,095.142 million

Total issued share capital

2,254,943,000 shares

Note: This document is not prepared for the purpose of soliciting investment. Investors are advised to make their own judgments when making investments. Please refer to the notes at the end of the final page of this document.

RaQualia Pharma's History and Future Growth

Feb. 2008
Establishment

Jul. 2008
Spin-out
from Pfizer

Jul. 2011
Initial public offering
JASDAQ: 4579

2014-2015
Drug Discovery and
Research Division relocated
to Nagoya University

2017
Launched two pet
phamaceuticals

2019
Launched human
pharmaceutical

Establis
hment

Growth

Develop
ment

Further
Growth