Kubota Pharmaceutical Holdings FY2018Q1 Update

May 17, 2018

Kubota Pharmaceutical Holdings is committed to translating innovation into a diverse portfolio of drugs and devices to preserve and restore vision for millions of people worldwide.

Cautionary Statement Regarding Forward Looking Statements

This presentation contains forward-looking statements concerning our product development, our ability to successfully commercialize our product candidates, our technology, our competitors, our intellectual property, our financial condition and operating results and our plans for research and development programs and the timing thereof that involve risks, uncertainties and assumptions. Such forward-looking statements typically can be identified by the use of words such as “expect,” “estimate,” “anticipate,” “forecast,” “intend,” “project,” “target,” “plan,” “believe” and similar terms and expressions. These statements are based on the current estimates and assumptions of the management of Kubota Pharmaceutical Holdings Co., Ltd. as of the date of this presentation and are subject to uncertainty and risks in circumstances, including, but not limited to the risk that our product candidates will not demonstrate the expected benefits and will not achieve regulatory approval or be successfully commercialized, the risk of delays in our ongoing or expected clinical trials, the risk that new developments in the intensely competitive ophthalmic pharmaceutical and device markets require changes in our clinical trial plans or limit the potential benefits of our product candidates, the accuracy of our estimates of the size and characteristics of the markets that may be addressed by our product candidates, the risk that our pre-clinical development efforts may not yield additional product candidates, and other risks and uncertainties inherent in the process of discovering and developing therapeutics and devices that demonstrate safety and efficacy. Given these uncertainties, you should not place undue reliance upon these forward-looking statements. Such forward-looking statements are subject to risks, uncertainties, assumptions and other factors that may cause our actual results to be materially different from those reflected in such forward-looking statements.

Important factors that could cause actual results to differ materially from those indicated by such forward-looking statements include, among others, those set forth in our reports on file with the Tokyo Securities Exchange and the United States Securities and Exchange Commission. The Company does not undertake any obligation to release publicly any revisions to these forward-looking statements to reflect events or circumstances after the date hereof or to reflect the occurrence of unanticipated events. All statements contained in this presentation are made only as of the date of this presentation.

Company Overview and Development Strategy

Kubota Pharmaceutical Group Vision

Our goal is to preserve and restore vision for millions of people suffering from eye diseases worldwide.

- **Address unmet medical needs in sight-threatening diseases through scientific innovations**
- **Build a unique company offering continuous value creation to our society**
- **Create an positive working environment and enhance quality of life for employees**

Company Overview

An Ophthalmology-Focused, Science-Driven Company

People and Strategy

- Executive leadership with experience in health care management, life science administration & technology
- Broad-skilled employee base in R&D and operations with broad industry relationships

Business Development

- Quick Win – Fast Fail
- Translational Research –Initiate pre-clinical and clinical studies, and obtain proof of concept (POC) in human

Internal Research

- Continue to expand ophthalmic product pipeline through internal research and seek for in-licensing opportunities
- Establish a total solution in ophthalmology for drugs and devices

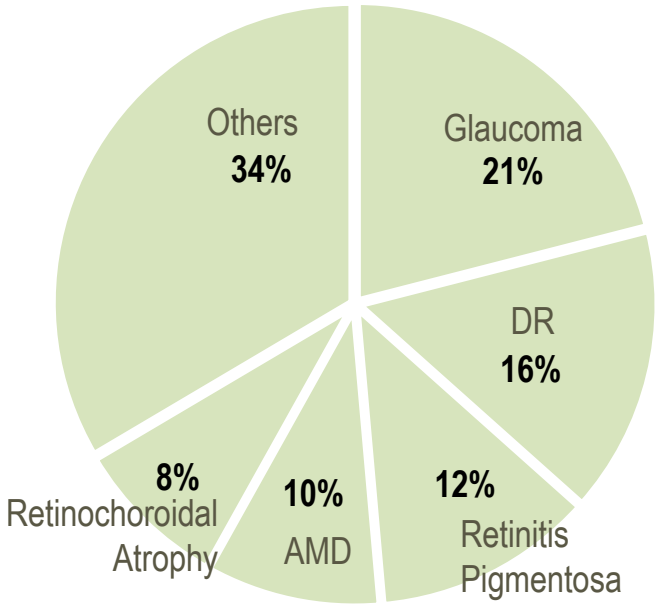
Partnership

- Partner with leading universities and research institutes for collaboration and in-licensing in the U.S. and Europe
- Enhance our technologies by partnering with large pharmaceutical companies in or outside of Japan

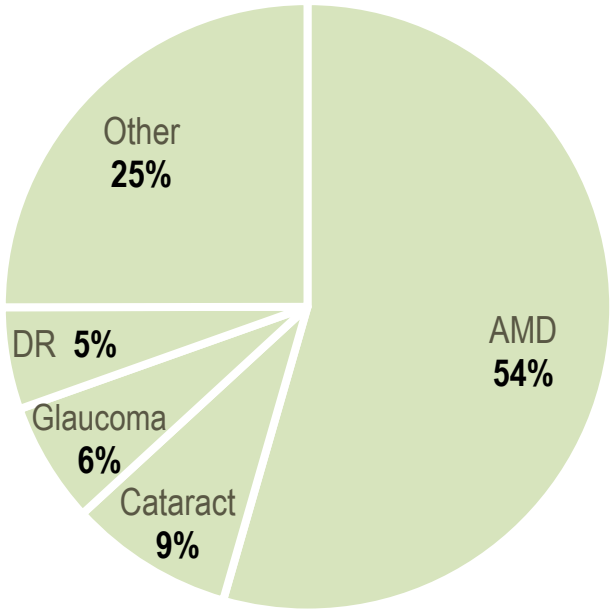
Leading Causes of Blindness in Japan, US and Europe

- **33 million people blind worldwide, and 191 million people suffer from moderate and severe vision impairment (MSVI)**

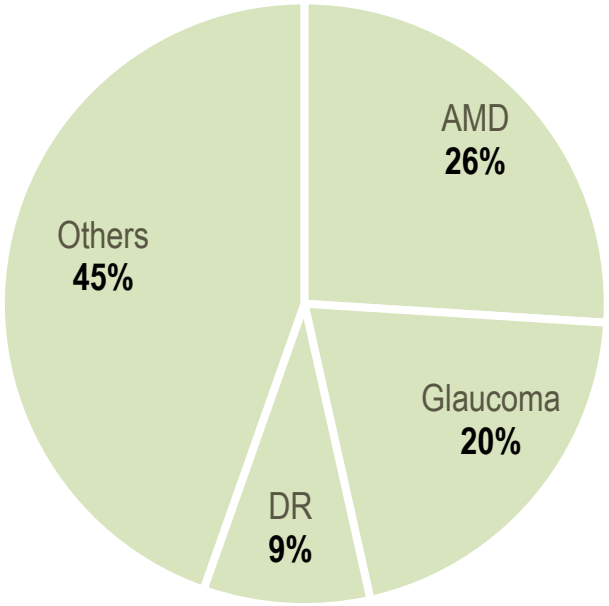
Japan[1]



Unites States[2]



Europe[3]



Source:

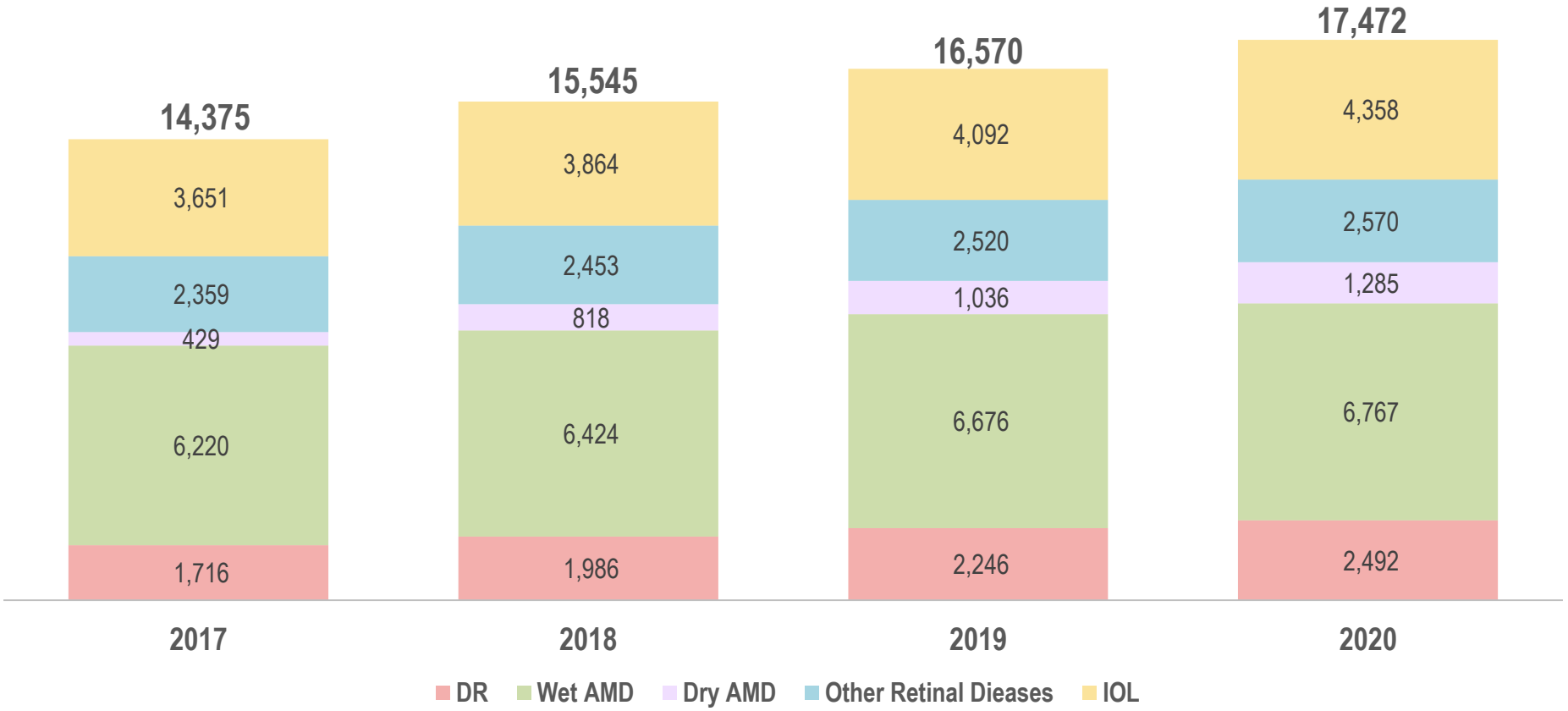
1) 厚生労働省 難治性疾患等克服研究事業「網膜脈絡膜・視神経萎縮症に関する調査研究 平成25(2013)年度」報告書 (A 2013 Report by Ministry of Health, Labour and Welfare)

2) Nathan C. et al. Causes and Prevalence of Visual Impairment Among Adults in the United States. *Arch Ophthalmol*122 (2004)

3) Kocur I, Resnikoff S. Visual Impairment and blindness in Europe and their prevention. *British Journal of Ophthalmology* 86, 716-722 (2002)

Retinal Diseases and IOL: Global Market Revenue Forecast

Global revenue forecast to reach $17.5B in 2020
CAGR 6.7% from 2017 – 2020

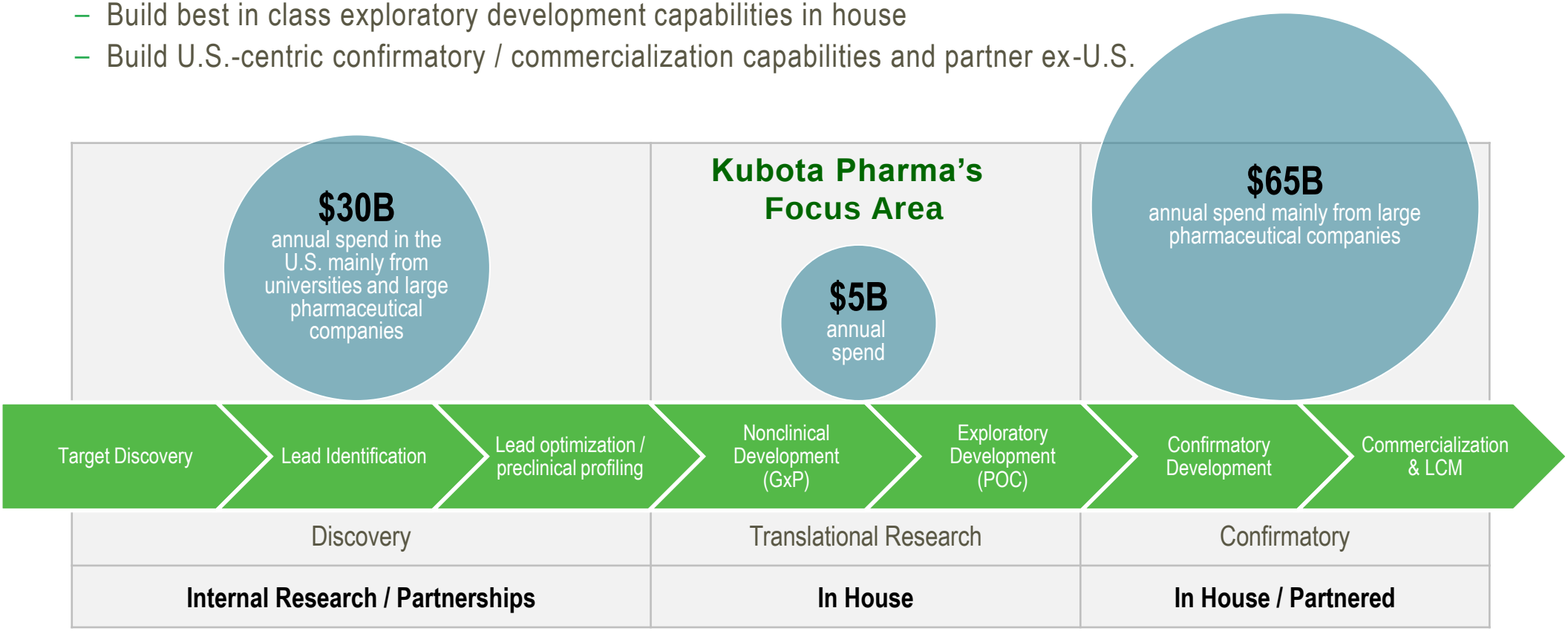


Sources:
Visiongain, Macular Degeneration (AMD) and Other Retinal Diseases: World Drug Industry and Market 2015-2025。
Market Scope, 2015 Comprehensive Report on the Global IOL Market。

（US$ in millions）

Business Development Focus

- **Kubota Pharma's business focus is to bridge the chasm between early discovery and late confirmatory stages, namely, the translational research stage.**
  - Partner with universities and leverage public funding in the discovery phase
  - Build best in class exploratory development capabilities in house
  - Build U.S.-centric confirmatory / commercialization capabilities and partner ex-U.S.



Source: Give to Cure 2015

Development Pipeline

Therapeutics

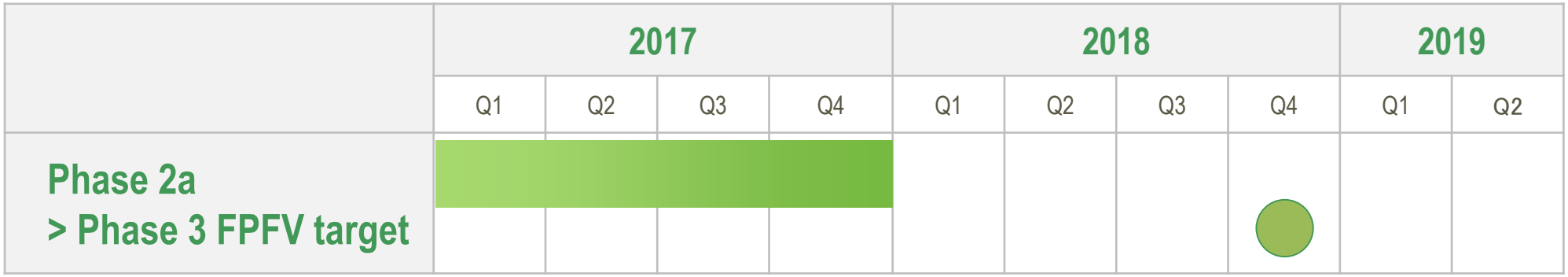
Program	Indications	Pre-clinical	Phase 1	Phase 2	Phase 3
Emixustat HCl	Proliferative Diabetic Retinopathy				
Emixustat HCl	Stargardt Disease				
Small Molecule	Cataract, Presbyopia				
Gene Therapy	Retinitis Pigmentosa				
Small Molecule	Diabetic Macular Edema, Wet AMD				

Devices

Program	Description	Design & Prototype	Clinical Trial & Product Eng.	Regulatory Approval [510(k)]
Remote Medical Monitoring Device	**Home-based miniature OCT** (optical coherence tomography)			

Stargardt Disease
using Emixustat Hydrochloride

Phase 2a Study of Emixustat for Stargardt Disease Complete; Moving to Phase 3



- **Multicenter (up to 6 clinical sites in the US), randomized, double-blind trial**
- **22 patients diagnosed with macular atrophy secondary to Stargardt randomly assigned to one of three treatment arms in a 1:1:1 ratio. Treatment arms include: emixustat 2.5 mg, 5 mg, and 10 mg. Subjects orally took study drug once daily in the evening for one month. Designed to evaluate the pharmacodynamics, safety and tolerability of emixustat in subjects.**
- **Evaluations**
  - Change in electrical response of the retina to a flash of light, as measured by electroretinogram
  - Percent suppression compared to baseline of rod b-wave amplitude recovery after a photobleaching light
- **Results**
  - ERG recovery after photobleaching was dose-dependent and a maximum reduction in recovery rate was more than 90% compared to baseline
  - Showed safety and tolerability of emixustat with the administered doses in subjects

2018 → **Planned initiation of Phase 3 clinical trial**

Phase 3 Study of Emixustat for Stargardt

In abca4-/-Mice models of Stargardt disease and retinal degeneration, emixustat was found to stop and reverse the accumulation of A2E and to preserve the integrity of the retina.

In Phase 2a study at US clinical sites, 22 patients diagnosed with macular atrophy secondary to Stargardt randomly took emixustat once daily. The results showed safety and tolerability of emixustat with the administered doses in subjects.

1

Received Orphan Drug Designation from the FDA in January 2017

Planned initiation of phase 3 clinical trial in Q4 2018

2

Seeking early approval in Japan, discussing with PMDA

Retinitis Pigmentosa
using Gene Therapy (Optogenetics)

Optogenetics Development Plan

Kubota Pharma intends to pursue Orphan Drug status to allow for potentially rapid development and extended regulatory exclusivity.

2016–2018	Pre-clinical development necessary to file an IND
2018–2019	Anticipated initiation of Phase 2 clinical study for patients with Retinitis Pigmentosa
2019	POC (Proof of Concept) Target

Abbreviation: IND, investigational new drug

Remote Medical Monitoring Device
Home-based Miniature OCT
(optical coherence tomography)

Clinical Study of PBOS

	2018			
	Q1	Q2	Q3	Q4
Clinical Trial				

Evaluating the quality and resolution of assessments taken with the PBOS system in 10 healthy subjects as well as in around 30 patients with neovascular retinal diseases at a clinical site in U.S.

Initiated clinical trial on March 23, 2018 (information disclosed on March 26, 2018)

Planning to complete by Q3 2018

<u>Evaluations (quality, resolution of assessment and safety)</u>

- **Demonstrate reproducibility and repeatability of retinal thickness measurements**
- **Demonstrate the correlation between retinal thickness**
- **Examine the PBOS device advantages and disadvantages, comparing to other high-cost OCT device**
- **Based on the results from this study, improve the device and design smaller handheld device**

PBOS Development Plan

Targeted for wet AMD, DME, and other neovascular retinal disease

2017–2018	Design and prototype
2018	Clinical trial and product engineering (initiated March 2018)
2019	Anticipated regulatory approval, 510(k)

Abbreviation: IND, investigational new drug

FY2018Q1
Financial Results

Overview of FY2018 Q1: P/L (IFRS)

¥ Millions	3 months ended March 31, 2017	3 months ended March 31, 2018	Inc/Dec	Reasons for change
Operating revenue	**—**	**—**	**—**	
Expenses	**1,080**	**751**	**-329**	
R&D	653	549	-104	
Internal Research	653	549	-104	• Increased efficiencies realized from prior year cost savings measures • decreased share-based compensation expense • The completion of Phase 2 PDR study
G&A	427	202	-225	• Lower personnel expenses due to cost savings measures - ¥ 120M • Completion of the IFRS project and the Redomicile Transaction：¥ -94M etc.
Loss from operations	**-1,080**	**-751**	**＋329**	
Net loss	**-1,038**	**-696**	**＋342**	

Overview of FY2018 Q1: B/S (IFRS)

¥ Millions	Dec 31, 2017	Mar 31, 2018	Inc/Dec	Reasons for change
Current assets	**11,673**	**9,670**	**-2,003**	
Cash and cash equivalents, and Other financial assets	11,197	9,260	-1,937	(*)
Other	476	410	-66	Amortization of prepaid clinical to fund further R&D research
Non-current assets	**1,724**	**2,252**	**＋528**	
Other financial assets	1,566	2,104	＋538	(*)
Other	158	148	-10	
Total assets	**13,396**	**11,922**	**-1,474**	
Current liabilities	**327**	**272**	**-55**	Decrease in Accrued compensation
Non-current liabilities	**103**	**95**	**-8**	
Equity	**12,967**	**11,554**	**-1,413**	Net loss and Cumulative translation adjustment
Liabilities and Equity	**13,396**	**11,922**	**-1,474**	
(*) Total Cash and cash equivalents, and Other financial assets	12,763	11,364	△1,399	Decrease primarily due to cash used in R&D activities and G&A cost, and JPY appreciation

Financial Outlook for FY2018*

¥ Millions	Operating revenue	Loss from operation	Loss before income tax	Net loss
FY2018 (Forecast)	—	-3,500	-3,370	-3,370
FY2017 (Actual)	—	-3,620	-3,445	-3,445

Exchange rate for the forecast: 1 US Dollar = \110

●Outlook for Operating revenue

- ➢ We are pursuing various partnering efforts and expects to generate revenue in the future through collaboration with strategic partners.

●Outlook for Loss from operations

- ➢ R&D cost will increase due to Phase 3 for Stargardt and continued development of PBOS and our other programs.
- ➢ G&A costs, including personnel and overhead expenses, will be lower than 2017 due to cost savings measures.

●Warrant Financing

- ➢ The 21st SARs with Moving-strike was issued in April 2018.
- ➢ Estimated total proceeds is \2.2 billion (Estimated amount is calculated based on the initial exercise price).

* Forward-looking financial information and estimates contained in this presentation were previously disclosed by the Company in the Company's Kessan Tanshin dated May 15, 2018. Such forward-looking financial information and estimates speak only as of the dates of initial disclosure and this presentation is neither updating nor confirming the previously provided forward-looking financial information, guidance and estimates.

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