



Kubota Pharmaceutical Holdings FY2018Q2 Update

August 21, 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.

“Acucela,” the Acucela logo and “Kubota” are among the registered trademarks or trademarks of Kubota Pharmaceutical Holdings in various jurisdictions.



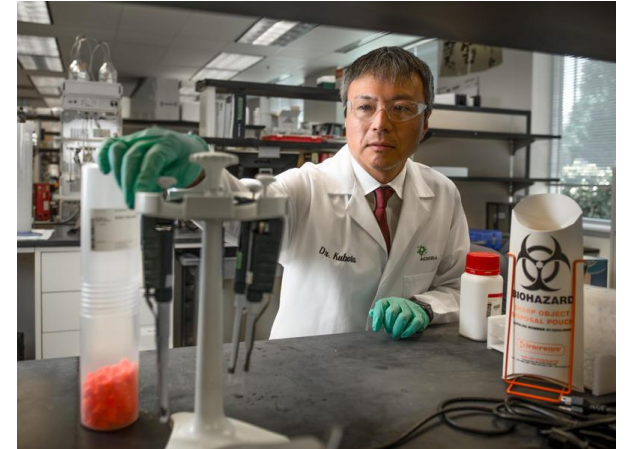


Company Overview and Development Strategy



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

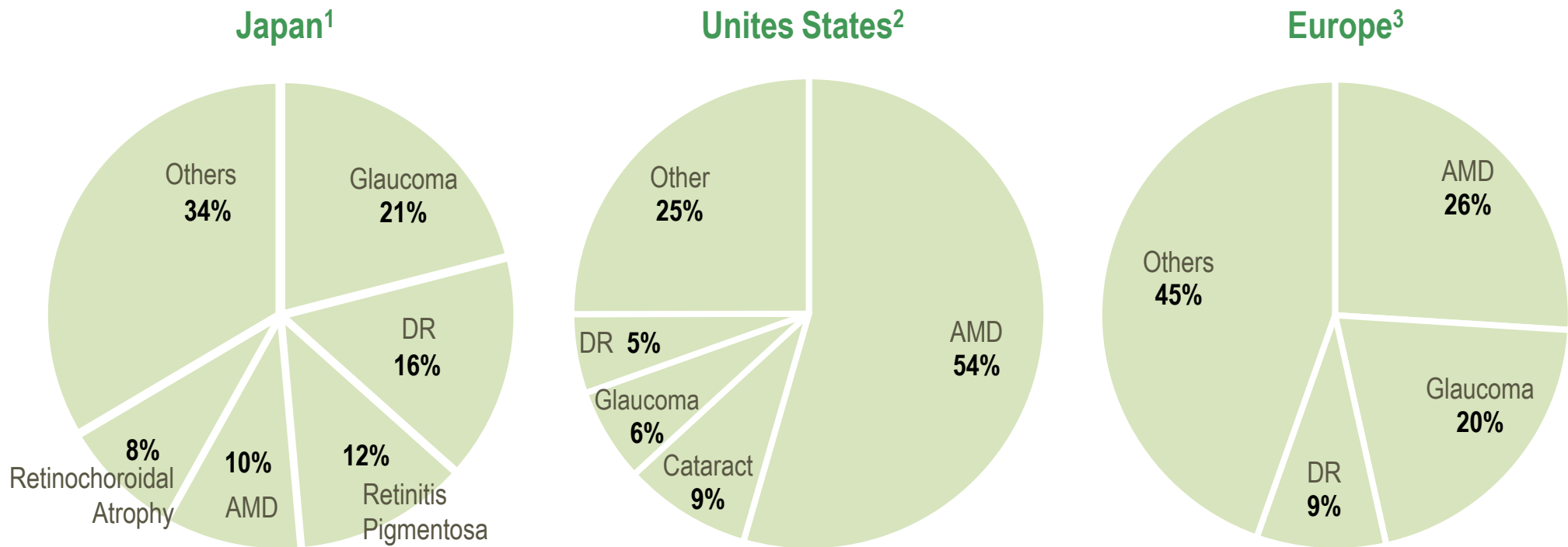
- Continue to expand ophthalmic product pipeline through internal research and seek for in-licensing opportunities
- Establish a total solution in ophthalmology for drugs and devices

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)



Source:

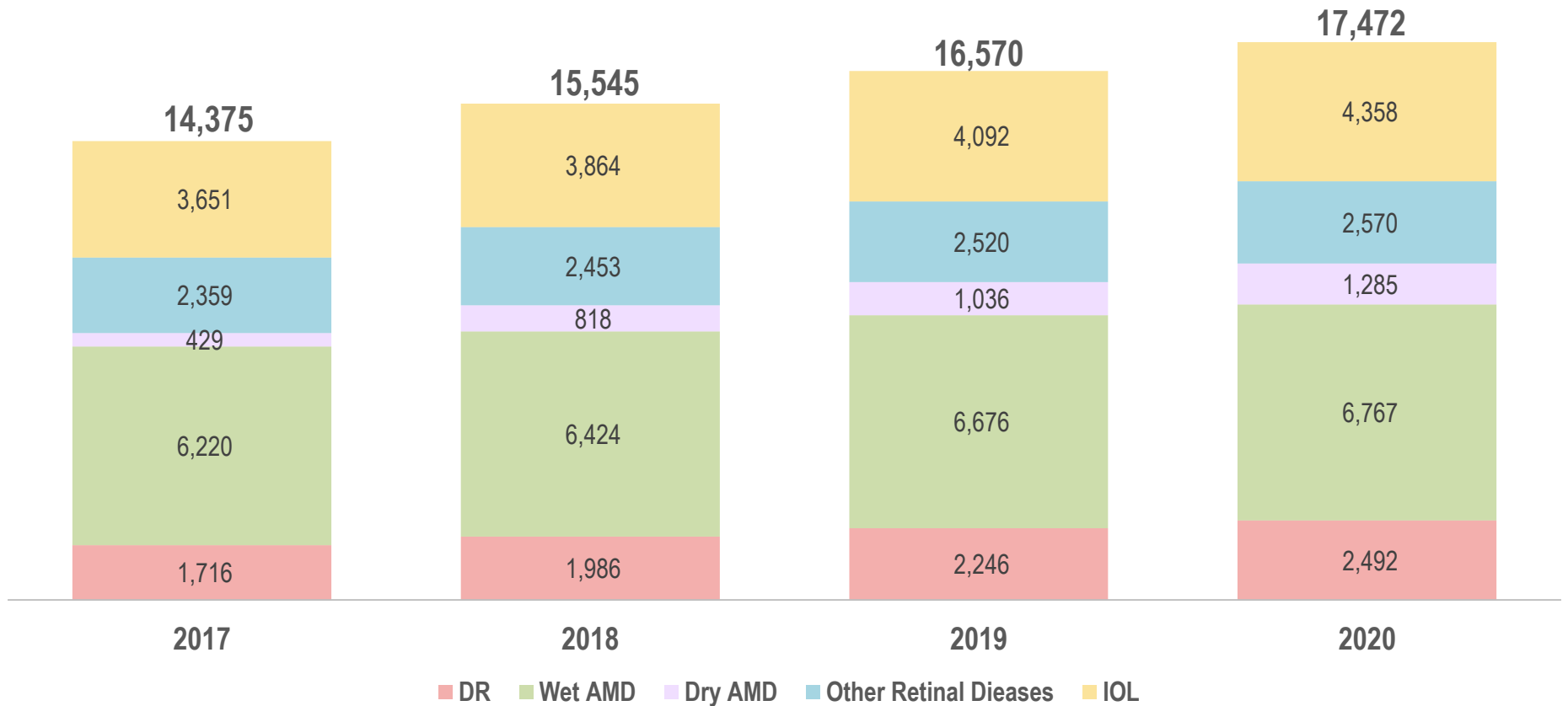
1) 厚生労働省 難治性疾患等克服研究事業「網膜脈絡膜・視神経萎縮症に関する調査研究 平成25(2013)年度」報告書 (A 2013 Report by Ministry of Health, Labour and Welfare)

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:

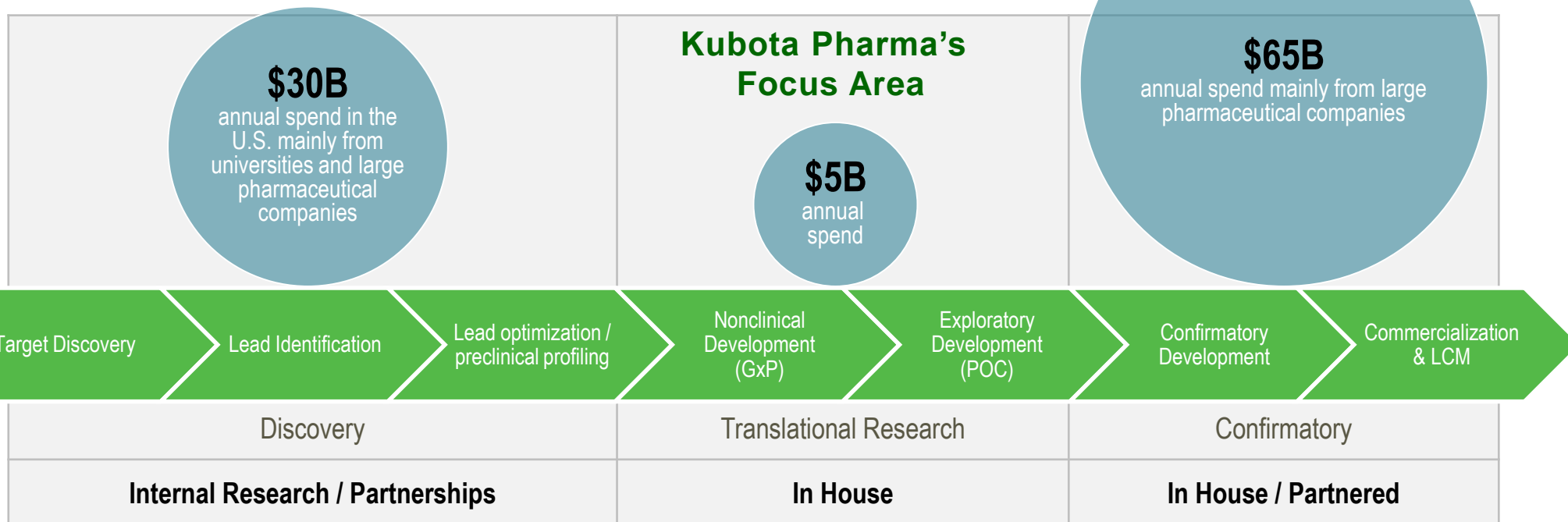
Visiongain, Macular Degeneration (AMD) and Other Retinal Diseases: World Drug Industry and Market 2015-2025.

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

Compounds	Indications	Pre-clinical	Phase 1	Phase 2	Phase 3	Target Market
Emixustat HCl	Proliferative Diabetic Retinopathy	████████████████████				Worldwide
Emixustat HCl	Stargardt Disease	████████████████████				Worldwide
Compound - 2	Cataract, Presbyopia	████				Worldwide
Compound - 3	Diabetic Macular Edema, Wet AMD	████				Worldwide

Gene Therapy	Indications	Pre-clinical	Phase 1	Phase 2	Phase 3	Target Market
Human Rhodopsin	Retinitis Pigmentosa	████				Worldwide

Device	Description	Design & Prototype	Clinical Trial & Product Eng.	Regulatory Approval [510(k)]	Target Market
Remote Medical Monitoring Device PBOS: Patient Based Ophthalmology Suite	Home-based miniature OCT (optical coherence tomography)	████████████████████			Worldwide



Stargardt Disease using Emixustat Hydrochloride



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

	2017				2018				2019	
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2
Phase 2a > Phase 3 FPFV target								●		

- **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





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)

Targeting completion during 2018

Evaluations (quality, resolution of assessment and safety)

- **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 monitoring

2018

Anticipated clinical trial completion, and product engineering and specification finalization

2018–2019

Anticipated production model build and evaluation, and regulatory approval (510(k))

2019–2020

Targeting commercial manufacturing readiness

Abbreviation: IND, investigational new drug



Retinitis Pigmentosa using Gene Therapy (Optogenetics)



Optogenetics Development Plan

2018–2019

Anticipated initiation of promoter and capsid enrichment, and transgene optimization programs

2020

Anticipated initiation of CNC process development and pre-IND nonclinical studies

2021

Targeting IND filing to initiate clinical study for patients with Retinitis Pigmentosa

Abbreviation: IND, investigational new drug



FY2018Q2 Financial Results



Overview of FY2018 Q2: P/L (IFRS)

¥ Millions	6 months ended June 30,		Inc/Dec	Reasons for change
	2017	2018		
Operating revenue	—	—	—	
Expenses	2,071	1,488	-582	
R&D	1,299	1,094	-205	<ul style="list-style-type: none"> • Increased efficiencies realized from prior year cost savings measures • Decreased share-based compensation expense • The completion of Phase 2 PDR study and Phase 2a Stargardt study.
G&A	771	394	-377	<ul style="list-style-type: none"> • Lower personnel expenses due to cost savings measures - ¥ 196M • Completion of the IFRS project and the Redomicile Transaction : ¥ -115M
Loss from operations	-2,071	-1,488	+582	
Net loss	-1,986	-1,384	+602	

Overview of FY2018 Q2: B/S (IFRS)

¥ Millions	Dec 31, 2017	Jun 30, 2018	Inc/Dec	Reasons for change
Current assets	11,673	9,842	-1,830	
Cash and cash equivalents, and Other financial assets	11,197	9,463	-1,734 (*)	
Non-current assets	1,724	2,292	+ 568	
Other financial assets	1,566	2,136	+ 570 (*)	
Total assets	13,396	12,135	-1,262	
Current liabilities	327	368	+ 42	Increase in Accrued liabilities and Trade payables
Non-current liabilities	103	93	-10	
Equity	12,967	11,674	-1,293	- Decrease due to Net loss - Increase due to the Warrant exercise
Liabilities and Equity	13,396	12,135	-1,262	
(*) Total Cash and cash equivalents, and Other financial assets	12,763	11,599	-1,164	- Operating CF: ¥ -1,279M - Warrant exercise of 780K shares: \ +314M

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 Japanese Yen

There are no changes to the earnings projections for FY2018 released on February 13, 2018.

● 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 August 14, 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.



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.

