

Mitsubishi Tanabe Pharma Corporation  
Business Briefing



Mitsubishi Tanabe Pharma

# *Open Up the Future*

December 5, 2017

**Masayuki Mitsuka**  
President and Representative Director

## Agenda

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### ▶ Medium–Term Management Plan 16–20 Fiscal 2017 Results and Progress

### ▶ Strategic Priority 3: Accelerating U.S. Business Development

- First Step in U.S. Business  
Radicava: Current Status and Future Initiatives
- Expanding U.S. Business  
NeuroDerm's Pipeline
- Sustained Expansion in U.S. Business  
Enhancing U.S. Pipeline

### ▶ Strategy Priority 2: Strengthening IKUYAKU and Marketing

- Autoimmune diseases field
- Diabetes / kidney diseases field
- Fiscal 2017 Topics  
Canalia

### ▶ Strategic Priority 1: Maximizing Pipeline Value

- Status of pipeline  
Fiscal 2017 Topics
- Principal pipeline items and approval targets

### ▶ Strategic Priority 4: Reforming Operational Productivity

### ▶ Targeting the Objectives of the Medium–Term Management Plan 16–20

### ▶ Initiatives to Increase Corporate Value

- The Social Value that We Are Aiming to Create

# Medium–Term Management Plan 16–20 Fiscal 2017 Results and Progress

# Principal Results and Progress with Four Strategic Priorities

Fiscal 2020 Objectives Revenue ¥500 billion Core operating profit ¥100 billion

## 1 Maximizing Pipeline Value

Late-stage drug candidate objective (including in-licensed candidates) 10 candidates

R&D investment (During the period of the medium-term management plan) ¥400 billion

### Principal results and progress

- 5 candidates progressing to late stage development trials
  - ✓ MT-2271 (seasonal influenza VLP) Phase3
  - ✓ MT-6548 (renal anemia) Phase3
  - ✓ MT-5547 (osteoarthritis) Phase2/3
  - ✓ MT-5199 (tardive dyskinesia) Phase2/3
  - ✓ ND0612 (Parkinson's) Phase3(Preparations under way)
- Enhancing our pipeline in the field of autoimmune diseases
  - ✓ Acquisition of STNM01 (Stelic Institute & Co.)

## 2 Strengthening IKUYAKU and Marketing

Domestic revenue objective (fiscal 2020) ¥300 billion

New drug and priority product revenue ratio 75%

Priority disease areas  
Autoimmune | Diabetes and kidney | Central nervous system | Vaccines

### Principal results and progress

- Domestic revenue (fiscal 2017 plan): ¥315.4 billion
- New drug and priority product revenue ratio: 70%
- Priority disease areas
  - ✓ Growth of Simponi, Tenelia, Canaglu
  - ✓ Approval / launch of Canalia
  - ✓ Launch of Rupatadine

## Principal Results and Progress with Four Strategic Priorities

### 3 Accelerating U.S. Business Development

U.S. revenue objective  
(Fiscal Year 2020)      ¥80 billion

U.S. strategic  
investment  
(During the period of  
the medium-term  
management plan)      More than  
¥200 billion

#### Principal results and progress

- **Launch of Radicava**
  - ✓ Solid start
  - ✓ Revenue of ¥1.1 billion recorded as of the end of September
  - ✓ Cumulative number of patients who have used Radicava: 1,200 (as of the end of November)
- **Acquisition of NeuroDerm**
  - ✓ Enhancing the pipeline in neurology in the U.S.
  - ✓ Acquisition procedures completed in October, became wholly owned subsidiary (total cost approximately ¥120.0 billion)

### 4 Reforming Operational Productivity

Cost of sales / SG&A  
expense reduction  
objective (Fiscal Year  
2020)      Vs. fiscal 2015  
¥20 billion

Number of employees  
(Fiscal Year 2020)      Consolidated domestic  
workforce  
5,000 employees

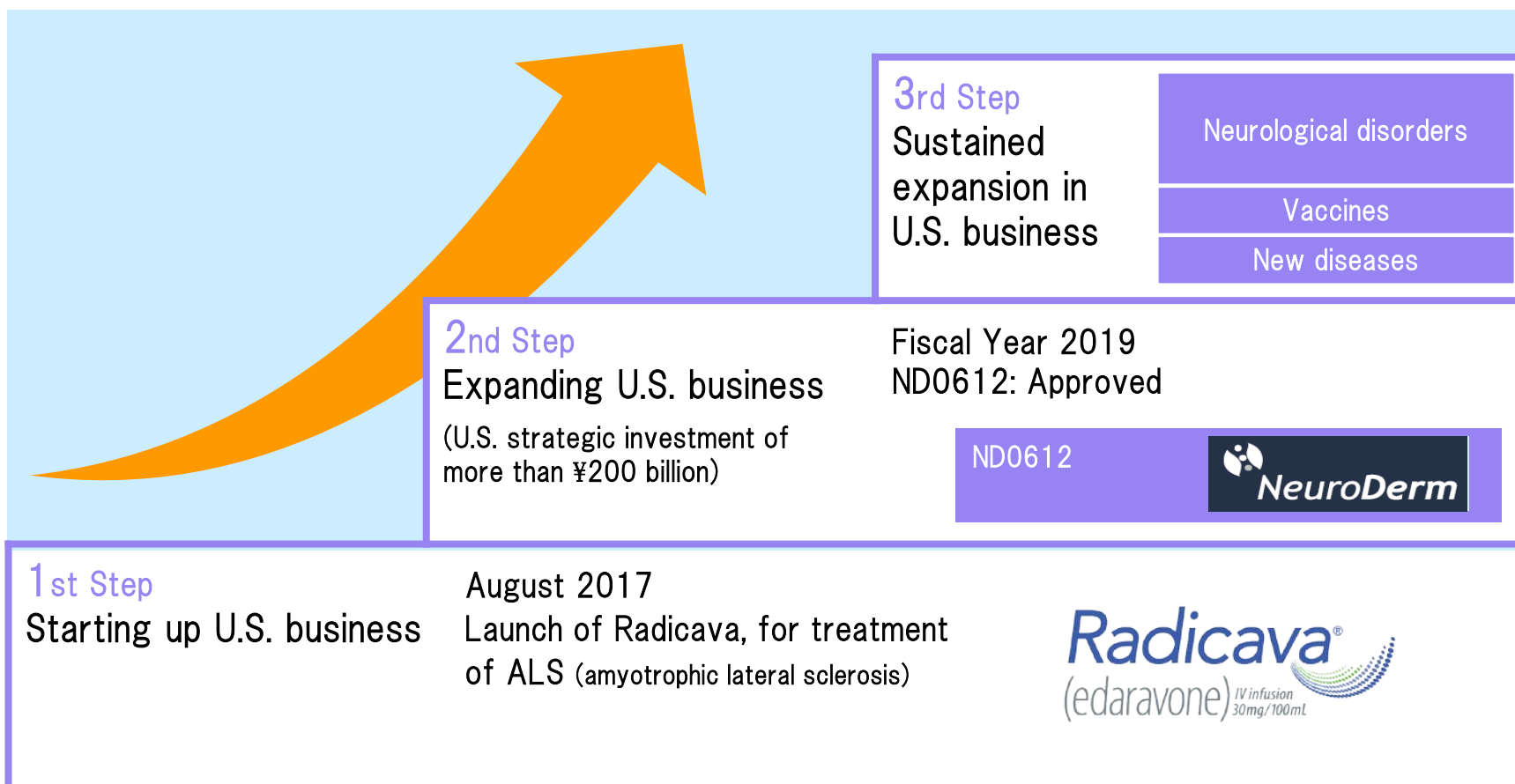
#### Principal results and progress

- Estimate of ¥3.0 billion in cost of sales and ¥7.0 billion in SG&A expenses (vs. Fiscal Year 2015)

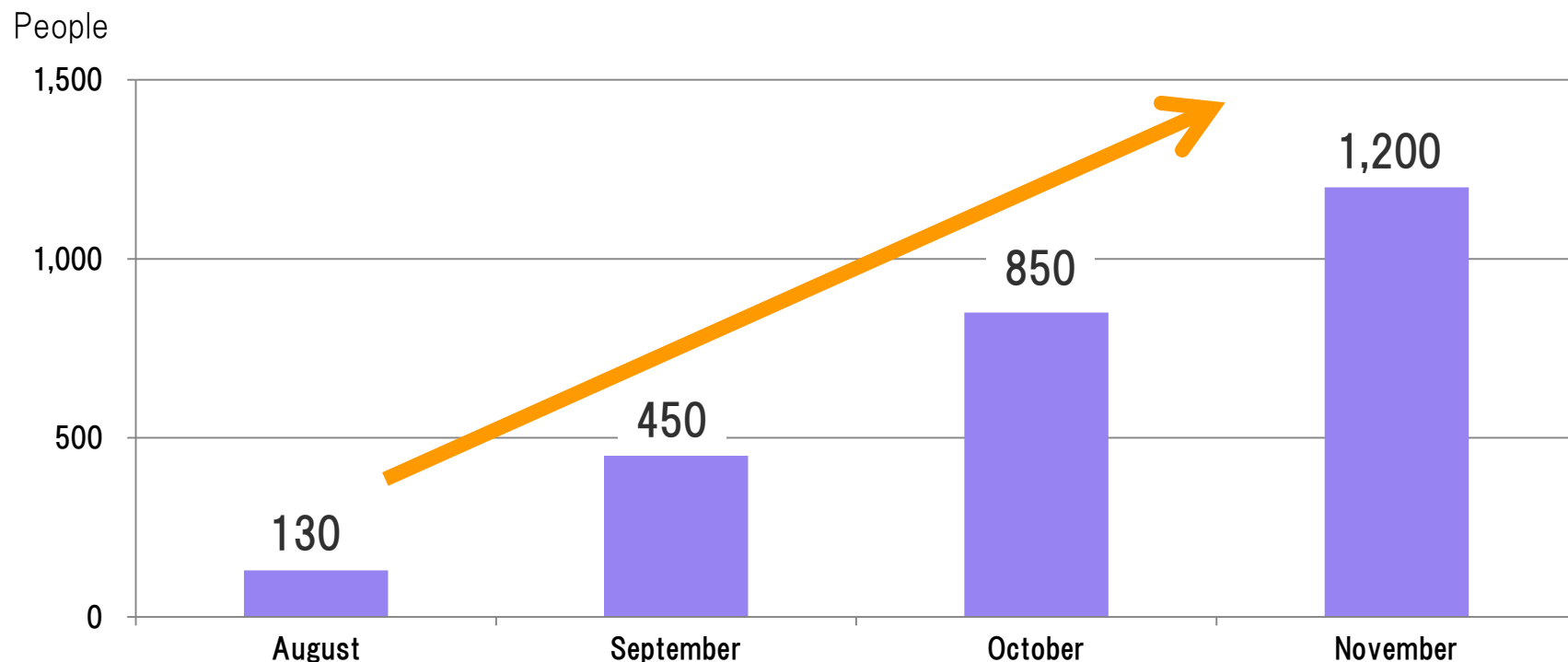
Strategic Priority 3  
Accelerating U.S. Business Development

## Roadmap for Accelerating U.S. Business Development

Make U.S. Business MTPC's second business pillar (following domestic market) by achieving revenue of ¥80 billion in Fiscal Year 2020 with sustained growth



## Number of Patients on Radicava (Cumulative)



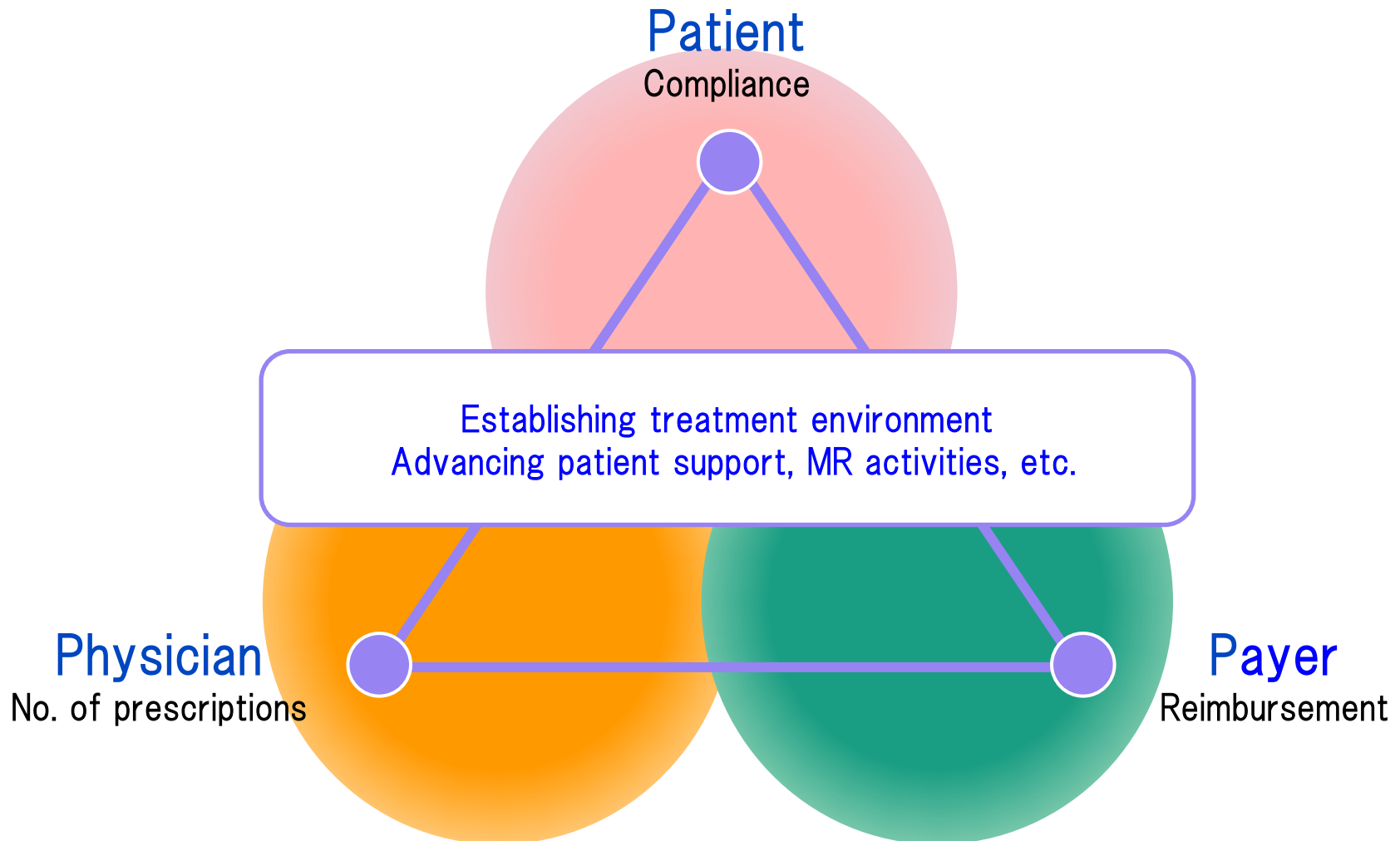
### Amyotrophic lateral sclerosis (ALS)

- ▶ Approximately 20,000 patients in the U.S.; about 5,000 to 6,000 patients diagnosed with ALS each year
- ▶ Approximately 10,000 patients in Japan. As of the end of November 2017, Radicut (approved in June 2015) had been prescribed to 2,750 patients.

Sources: Websites of the ALS Association and the Japan Intractable Diseases Information Center

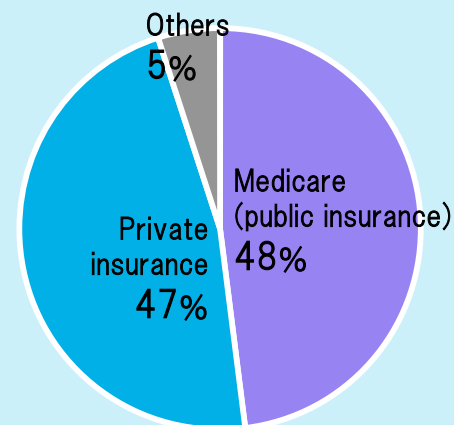
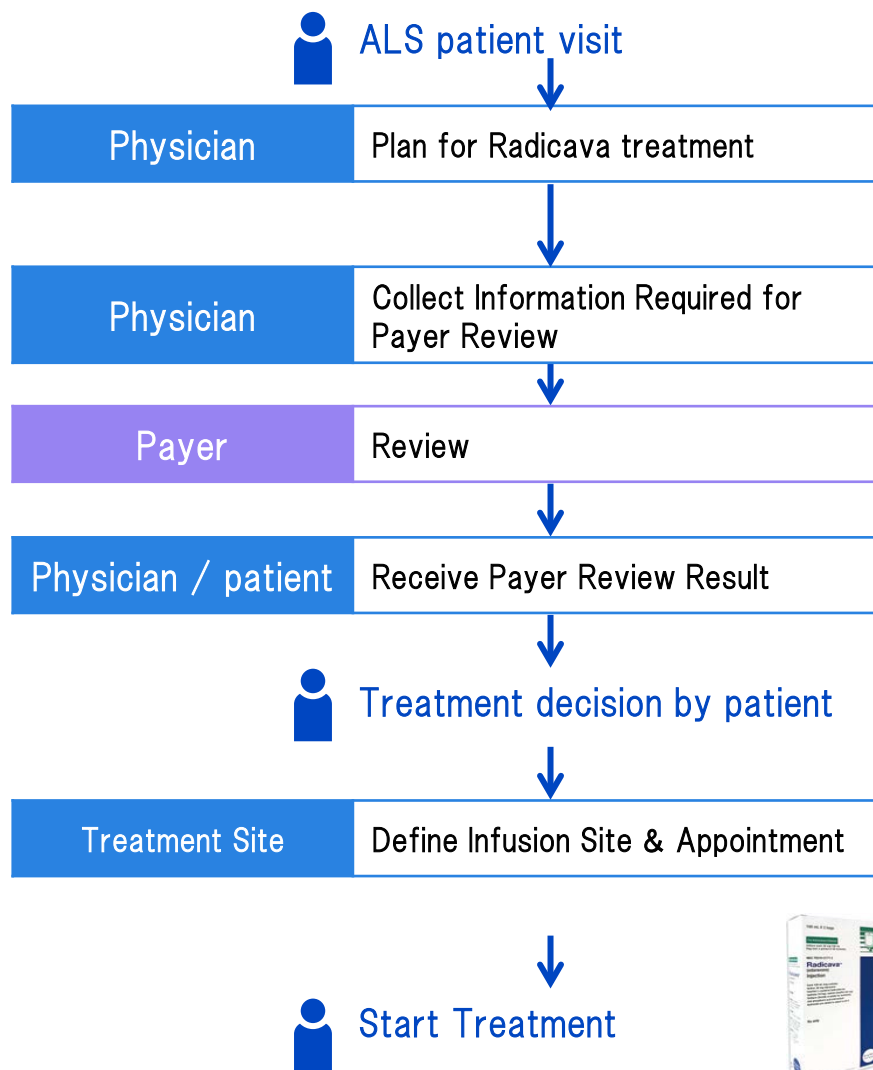


## 3P : Three core points for Radicava business

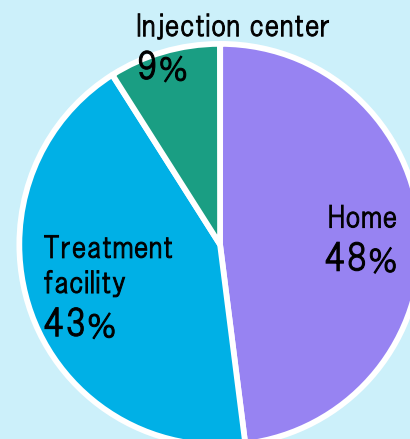




## Major steps to Radicava Infusion



Proportion as of November 2017



Proportion as of November 2017



## Patient Support

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### ▶ Information support: SearchLight Support

- Addressing questions from patients regarding insurance. We have smoothened the reimbursement process with the deployment of insurance reimbursement specialists.
- Providing information regarding treatment. We have established a hotline which provides information such as facilities that can infuse Radicava, to satisfy individual patient needs with detailed response and follow-up.

### ▶ Cost support: Bridge Program, Co-Pay Support Program

- **Bridge Program**

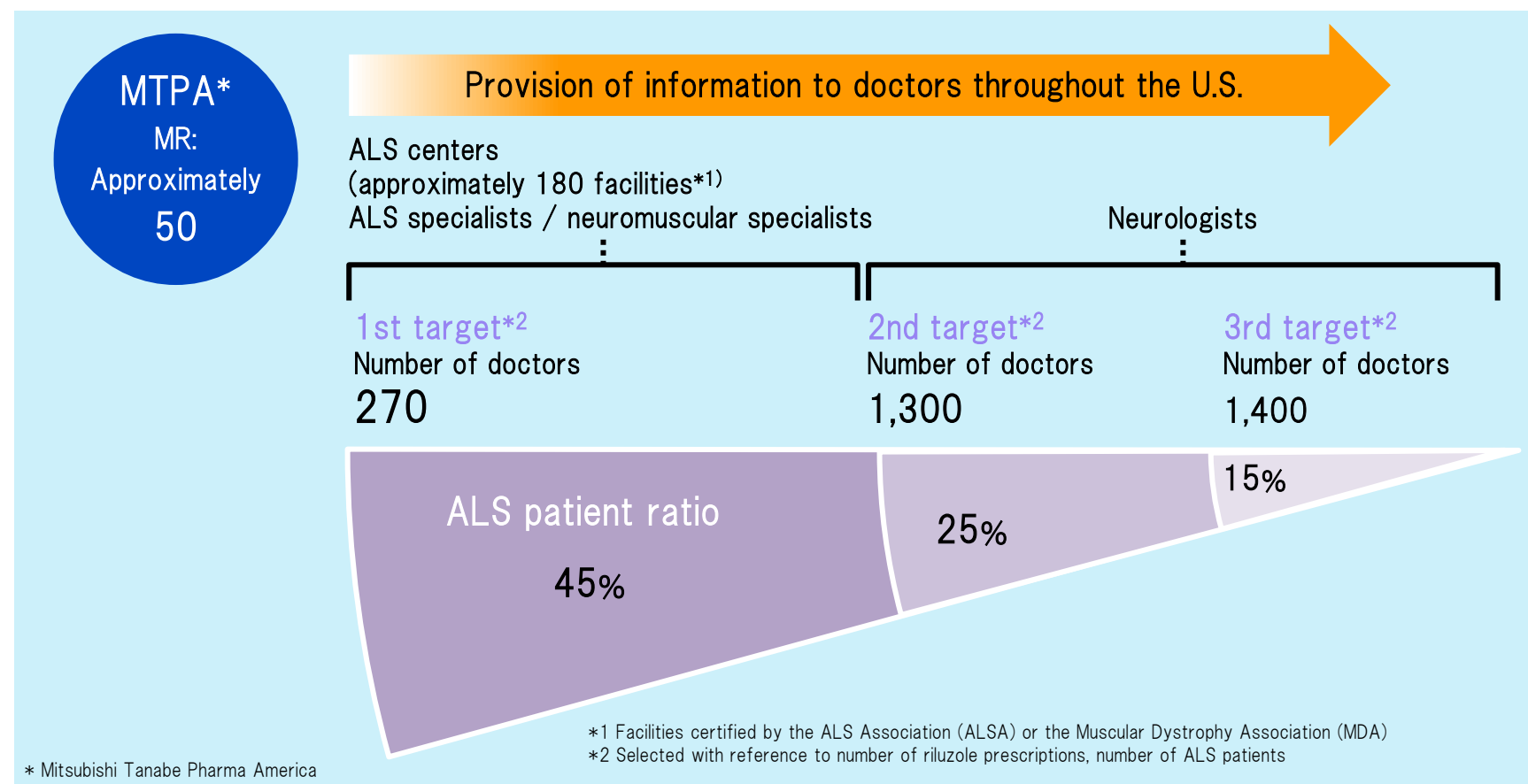
In case the payer review process will require some time, this program provides support to patients who want to use Radicava quickly. (Radicava would be provided free of charge for two months)

- **Co-Pay Support Program**

Support out of pocket fee for patients enrolled in private insurance (upper limit of \$20,000 per year).

## MR Activities

Initiated activities such as providing information focusing on ALS Centers (ALS specialists, neuromuscular specialists) for the 3 months post approval. Currently starting to reach out to neurologists as well.



## Maximizing the Value of Radicava

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### ▶ Business regions (indication: ALS)

- Japan, South Korea, U.S.: Launched
- Canada / Switzerland: Plan to file application in Q4 fiscal 2017
- Europe: Consulting with regulatory authorities
- ASEAN, others: Under consideration

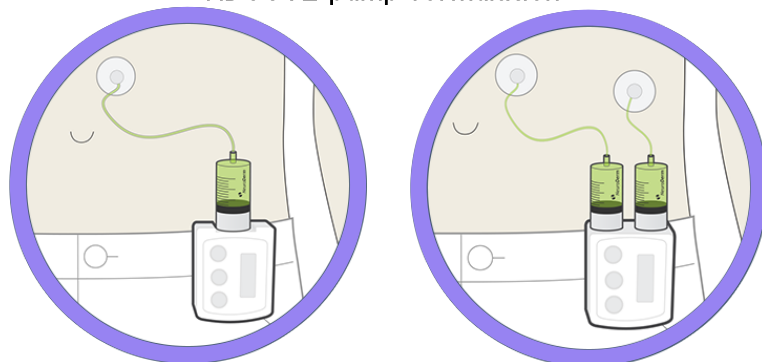
### ▶ LCM (Life Cycle Management)

- Application for 60 mg intravenous infusion bag formulation (plan to launch in fiscal 2019)
  - Increasing convenience with change from 2 bags per administration to 1 bag per administration
- Developing non-injection formulation
  - Reducing burden on ALS patients and caregivers, increasing convenience

# Introduction to NeuroDerm's Pipeline

Product name	Generic name	Expected indications	Stage	Approval timing
ND0612	Levodopa (LD) / carbidopa (CD) Long-acting subcutaneous injection administration pump / patch formulation	Parkinson's (moderate / severe)	Phase 3 Preparations under way	Fiscal 2019
ND0701	Apomorphine Continuous, subcutaneously delivered formulation administered via a pump	Parkinson's (severe)	Phase 1	—
ND0901	Levodopa prodrug / carbidopa New device for long-acting subcutaneous injection administration	Parkinson's (moderate / severe)	Phase 1 Preparations under way	—
ND0801	Nicotine / opipramol Transdermal	Cognitive disorders associated with central nervous system diseases	Phase 2	—

ND0612 pump formulation



ND0612 patch formulation (device under development)



## Positioning of NeuroDerm Drug Candidates

### General clinical course of Parkinson's



#### Number of Parkinson's patients

U.S.: approximately 1 million  
Europe: more than 1.2 million

\* Motor complications occur in about 50% of patients with moderate symptoms or severe symptoms (internal investigation).

#### Oral / percutaneous drug therapy

#### NeuroDerm drug candidates

Stabilizing blood levodopa concentration /  
delaying or avoiding surgery

Minimally invasive, continuous dopamine stimulation treatment  
(continuous subcutaneous delivery via a pump, etc.)

ND0612(LD/CD)

ND0701(apomorphine)

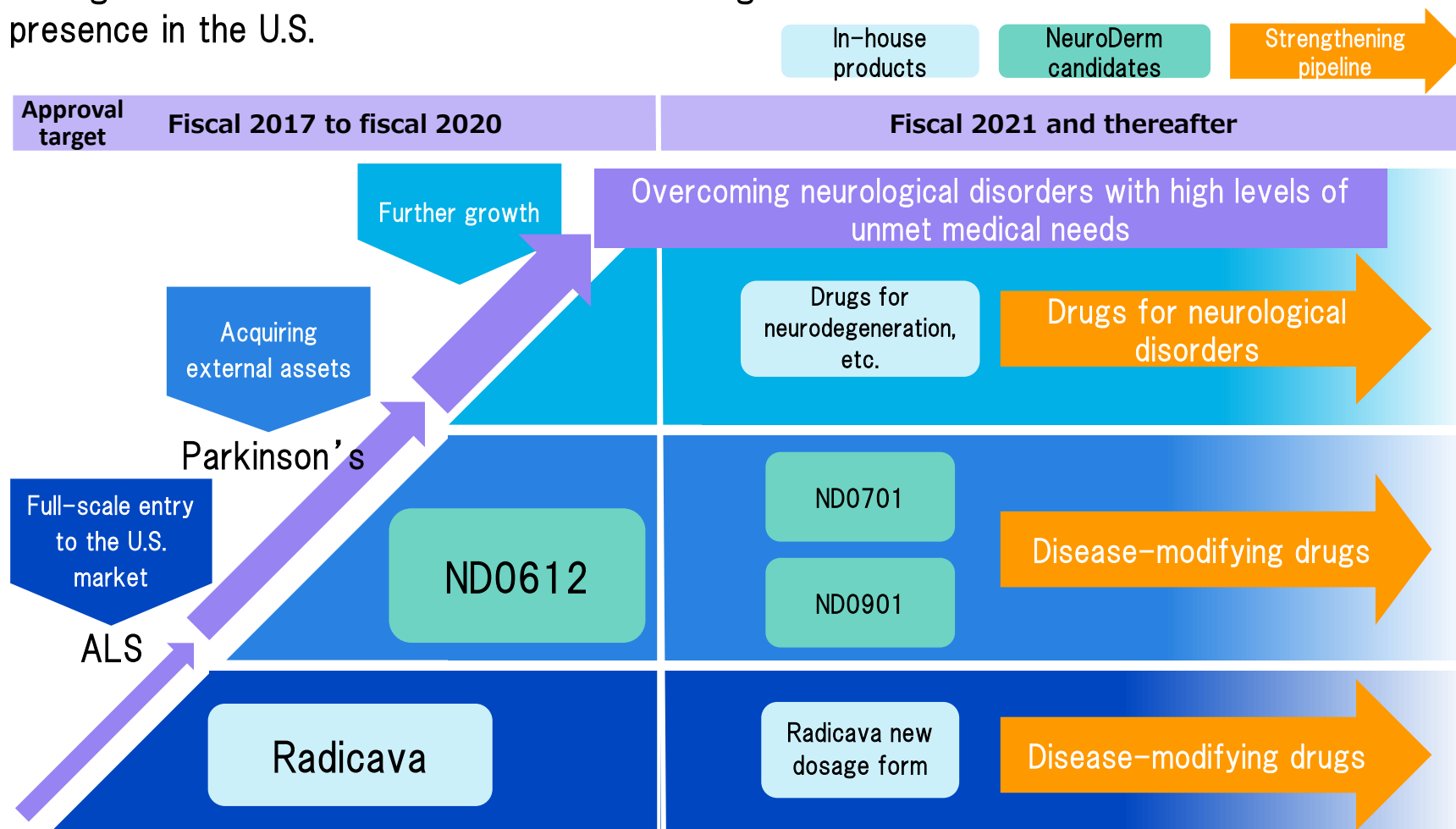
#### Treatments accompanying surgery

(deep brain stimulation and continuous intra-jejunal administration of levodopa through percutaneous endoscopic gastrostomy)

Source: Parkinson's Disease Foundation web site  
European Parkinson's Disease Association web site

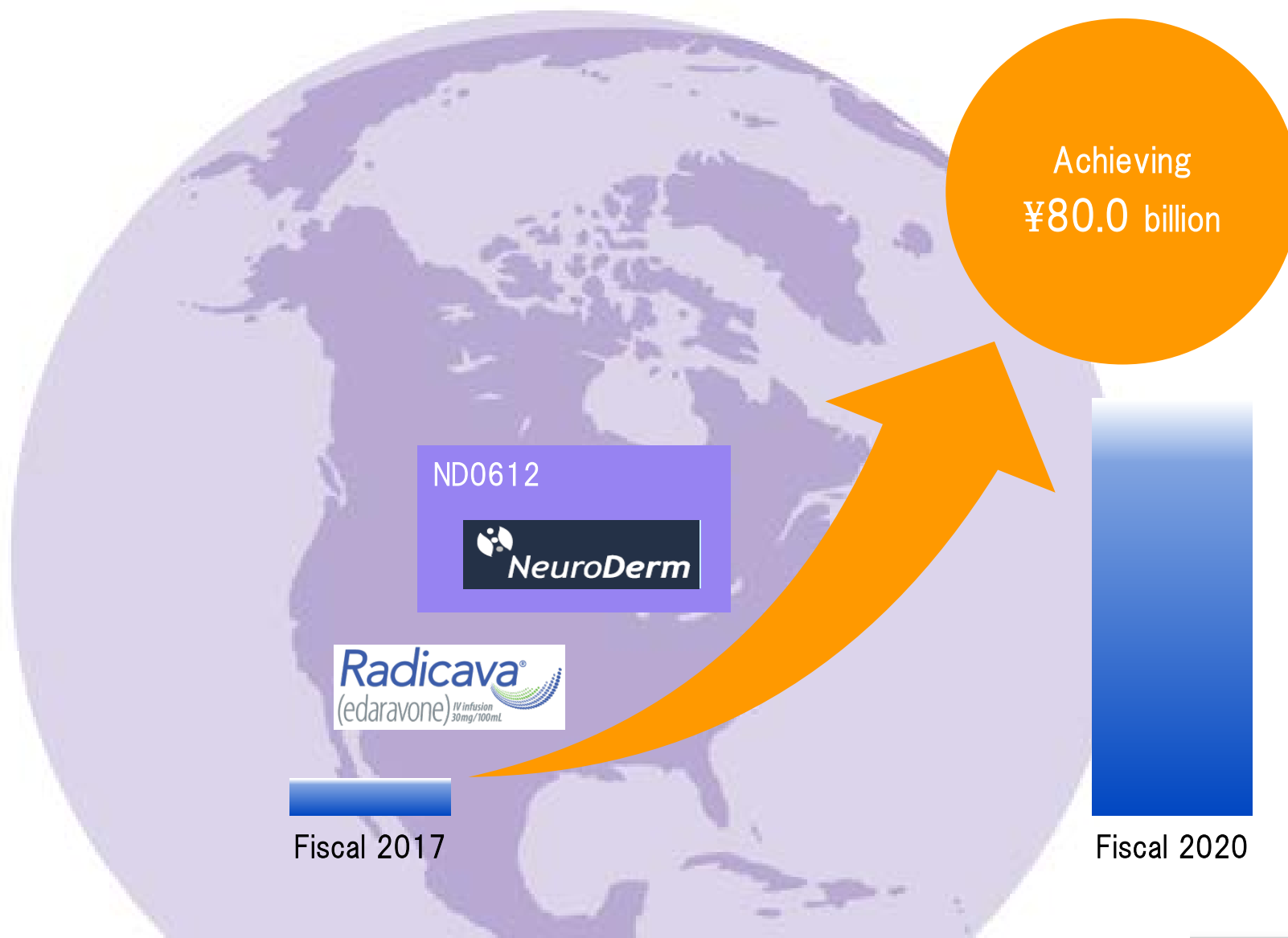
## Enhancing U.S. Pipeline

Targeting further growth centered on two diseases—ALS and Parkinson's—we will strengthen our initiatives in the field of neurological disorders and enhance our presence in the U.S.





## Outlook for Growth in Revenue in U.S. Business



Strategic Priority 2  
Strengthening IKUYAKU and Marketing

## Autoimmune Diseases Field

Priority products Remicade, Simponi, Stelara

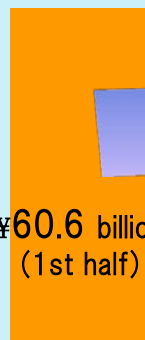
- ▶ Maintaining number one share in the field
- ▶ Aiming for the rapid launch of MT-5547

¥113.9 billion



Fiscal 2016

¥60.6 billion  
(1st half)



Fiscal 2017

New products



Fiscal 2020

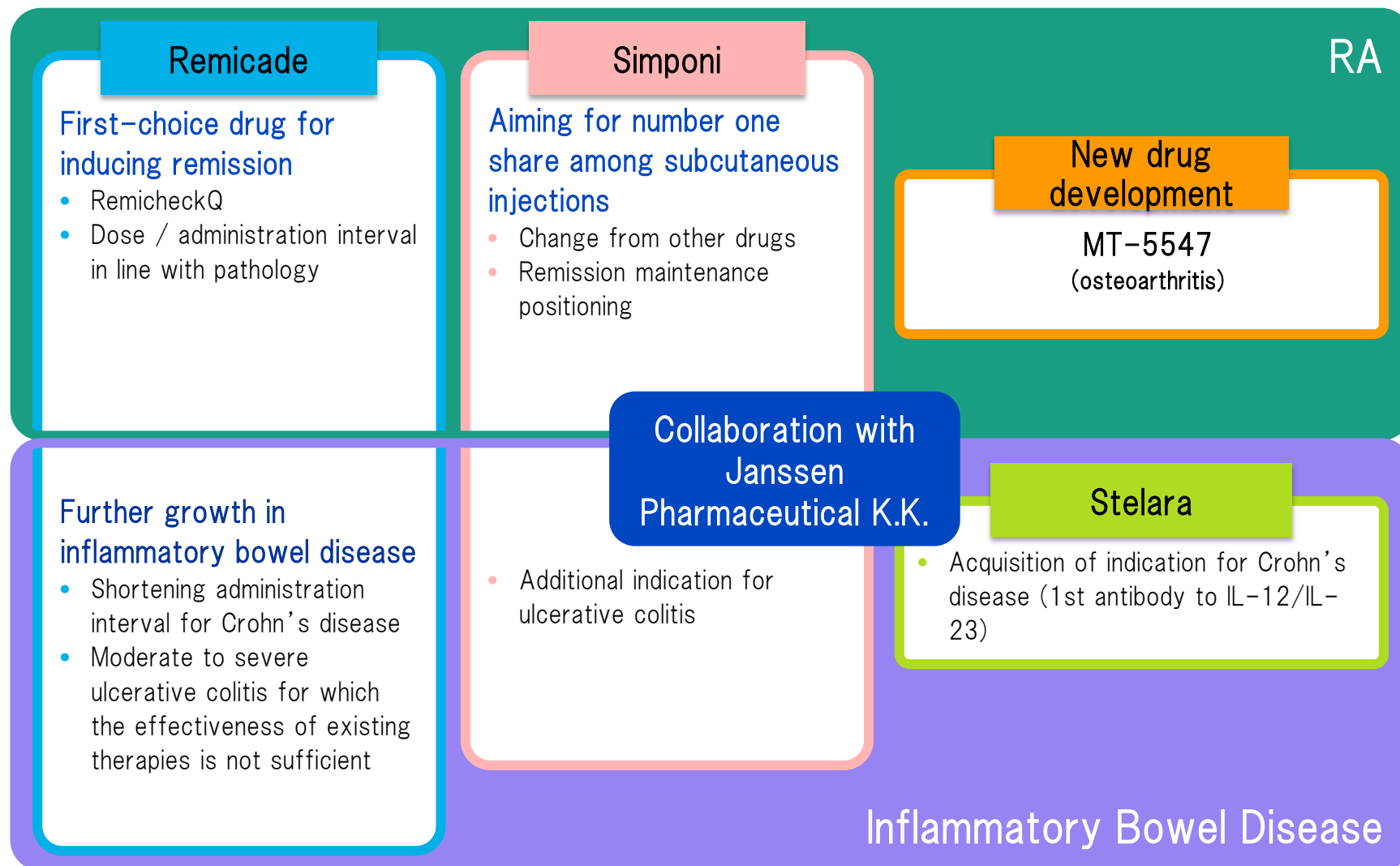
Future revenue target  
¥150 billion\*



Approximate  
expected revenue

\* Revenue is on an NHI drug price basis

## Autoimmune Diseases (RA / Inflammatory Bowel Disease)



## Diabetes and Kidney Diseases Field

Priority products Tenelia, Canaglu, Canalia

- ▶ Canalia launch
- ▶ Utilization of clinical research data (CANVAS / CREDENCE)
- ▶ Rapid launch of MT-6548
- ▶ Rapid achievement of additional indications for Canaglu

Future revenue target  
¥100 billion\*

Approximate  
expected revenue

Canaglu  
CANVAS/CANVAS-R: 10,000 subjects

Canalia  
(Tenelia+Canaglu combination)

CREDENCE

New products

Additional indications  
for Canaglu

¥33.2 billion

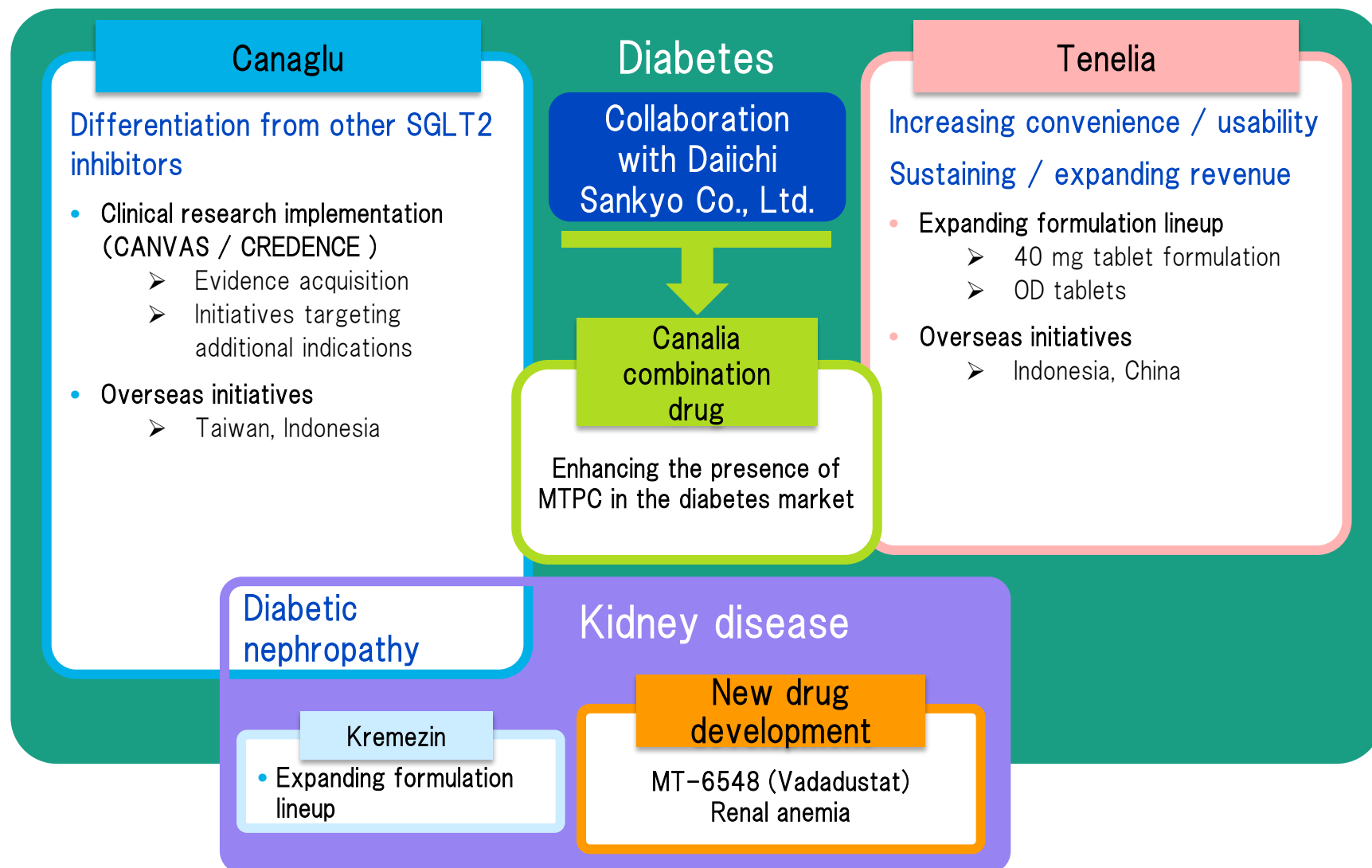
¥20.2 billion  
(1st half)

Fiscal 2016

Fiscal 2017

Fiscal 2020

\*Revenue is on an NHI drug price basis



## Features of Canalia Combination Tablets

### ▶ Japan's 1st combination drug including both DPP-4 inhibitor and SGLT2 inhibitor

- Launched September 2017
- Compared with combination therapy using each individual drug, the combination tablets will reduce the number of pills taken and the cost, and are expected to improve medication adherence and offer good blood glucose control.

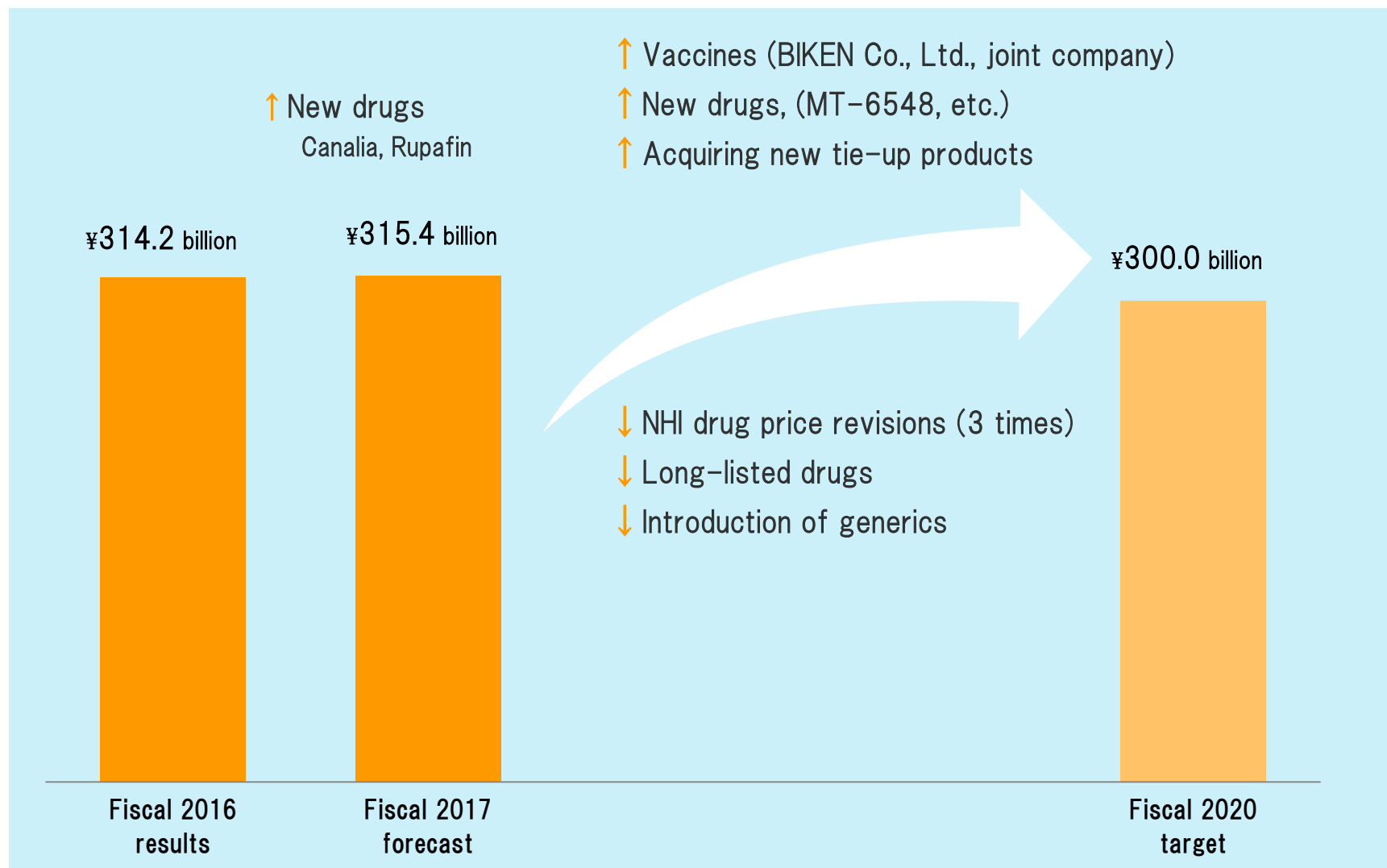


### Feedback from Key Opinion Leaders on the Medical Front Lines

- There is an issue with DPP-4 inhibitors of increased body weight leading to weakened effectiveness. Combination therapy with an SGLT2 inhibitor has a strong merit of a long-acting blood-glucose lowering effect.
- With the product name Canalia, the patients immediately understand what the combination tablets are, making it easy to provide patient compliance instruction.
- The Launch of Canalia will lead to prescriptions of Tenelia and Canaglu.



## Aiming for Revenue of ¥300.0 Billion in Fiscal 2020





Strategic Priority 1  
Maximizing Pipeline Value



# 10 Late-Stage Drug Candidates

Fiscal 2016 results

Fiscal 2017 plan

Fiscal 2017 results (end November)

10 late-stage drug candidates		Phase1	Phase 2 / POC trials	Late-stage development trials
Fiscal 2017: Initial plans		1 candidate	2 candidate	5 candidate
Autoimmune diseases	MT-1303	Japan: inflammation / autoimmunity	Europe: MS, PS, CD Japan: CD	→
	MT-5547			→ Japan: osteoarthritis
	MT-7117	Europe: dermatology, etc.	→	
	MT-2990	→ Europe: inflammation / autoimmune diseases, etc.		
Diabetes and kidney diseases	MT-6548		Japan: renal anemia	→ Japan: renal anemia
	MT-3995		Japan: NASH Japan, Europe: diabetic nephropathy	
Central nervous system diseases	MT-5199	Japan: tardive dyskinesia		→ Japan: tardive dyskinesia
	ND0612			U.S., Europe: Parkinson's (Preparations under way)
	MT-8554		→ U.S., Europe: Nervous system, etc.	
Vaccines Others	MT-2355			Japan: Combined 4 diseases + Hib
	MT-2271 Plant-based VLP vaccines			→ U.S., Europe, Canada, others: Seasonal influenza
	MT-4129	Europe: cardiovascular system, etc.		

In four fields, late-stage development trials are making favorable progress

Autoimmune diseases	<p><b>MT-5547:</b> fully human anti-NGF monoclonal antibody Generic name: fasinumab Start of phase 2/3 clinical trials in Japan for osteoarthritis</p>
Diabetes and kidney diseases	<p><b>MT-6548:</b> hypoxia inducible factor prolyl hydroxylase (HIF-PH) inhibitor Generic name: vadadustat Start of phase 3 clinical study in patients with anemia secondary to chronic kidney disease (renal anemia).</p>
Central nervous system diseases	<p><b>MT-5199:</b> vesicular monoamine transporter type 2 (VMAT2) inhibitor Generic name: valbenazine Start of phase 2/3 clinical study in Japan for tardive dyskinesia.</p>
	<p><b>ND0612:</b> Long-acting subcutaneous injection administration pump / patch formulation (levodopa / carbidopa) Clinical trials under way in Europe and the U.S. for Parkinson's</p>
Vaccines	<p><b>MT-2271:</b> Plant-based VLP (virus-like particle) vaccines Start of phase 3 clinical study in 7 countries, including the U.S., Europe, and Canada, for prophylaxis of seasonal influenza.</p>

#### Vaccine

#### MT-2271: Plant-based Seasonal Influenza VLP Vaccine

In August 2017, Medicago has initiated the Phase 3 clinical study in US, Europe, Canada and other countries

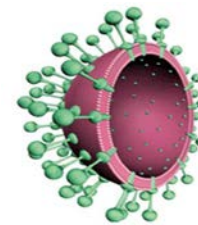
#### Study design of the Phase 3 clinical study

→ To evaluate the efficacy of VLP vaccine in 10,000 healthy adults

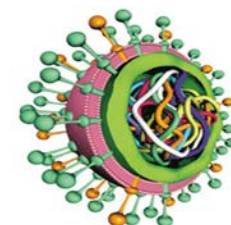
#### ► Phase 2 clinical study results

- Efficacy
  - The antibody response compares to that of licensed vaccines
  - Cell-mediated responses are higher than a standard dose of licensed vaccine
  - 30 µg per strain is the optimal dose
- Safety
  - Similar safety profile as licensed vaccines

➡ As a result of consultation with FDA about Phase 2 study results, Medicago has initiated the Phase 3 clinical study.



VLP



Influenza  
virus

## Drug candidates for which the strategy will change

### Autoimmune diseases

**MT-1303** (Sphingosine-1-phosphate receptor functional antagonist)

Generic name: amiselimod

Expected indications: ulcerative colitis, Crohn's disease, others

- In consideration of the business strategy and the market environment, including the competitive situation in the U.S., we will reevaluate the development plan for in-house development on our own for ulcerative colitis.

### Diabetes and kidney diseases

**MT-3995** (mineralocorticoid receptor antagonist)

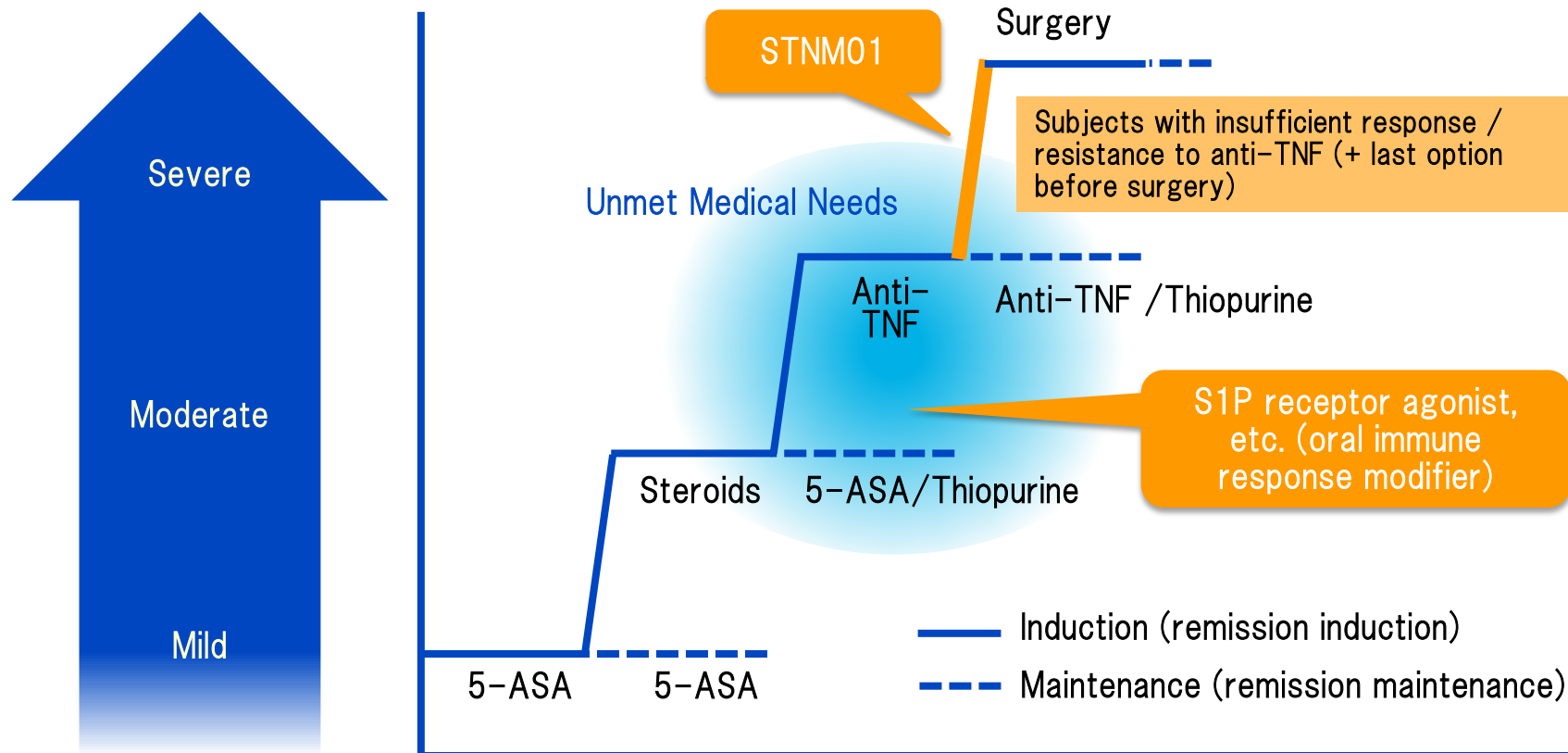
Expected indications: diabetic nephropathy, NASH (non-alcoholic steatohepatitis)

- Focusing on NASH, POC study currently under way. Expect to acquire top-line data in 1st half of fiscal 2019.

## Enhancing pipeline in priority areas

### Autoimmune diseases

Acquisition of nucleic acid pharmaceuticals “STNM01” (endoscopic injection) from Stelic Institute & Co., Inc. for ulcerative colitis and other diseases, will contribute to future initiatives in accelerating U.S. Business Development.



# 1 Maximizing Pipeline Value

## Principal pipeline items and approval targets

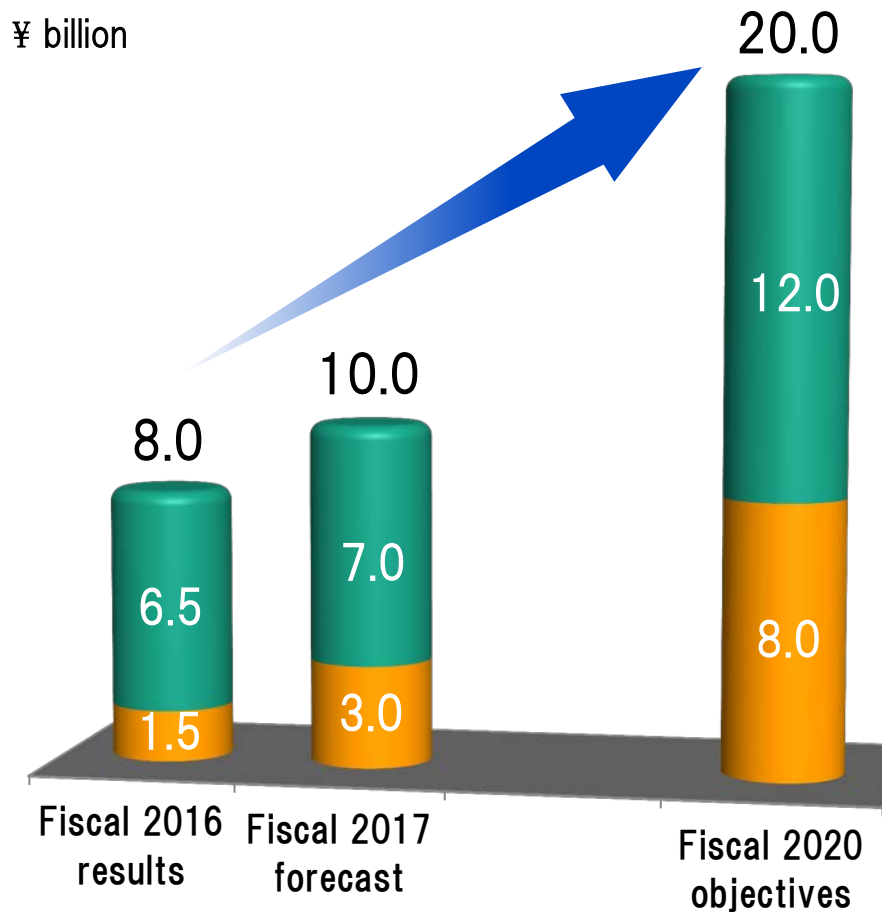
	2018	2019	2020	Fiscal 2021 and thereafter		
U.S. / Europe	MCI-186 ALS (Canada, Switzerland)	MCI-186 ALS (Europe)		MT-8554 Nervous system, etc.	ND0901 Parkinson's	ND0701 Parkinson's
		ND0612 Parkinson's		MT-7117 Dermatology	MT-2990 Inflammation / autoimmunity	MT-1303 Inflammation / autoimmunity
		MT-2271 Seasonal influenza VLP		MT-8972 H5N1 influenza VLP	MT-7529 H7N9 influenza VLP	
Japan			MT-5199 Tardive dyskinesia	MT-5547 Osteoarthritis	MT-3995 NASH	
			MT-6548 Renal anemia		Central nervous system diseases	
			MT-2355 Combined 4 diseases + Hib		Autoimmune diseases	
					Diabetes and kidney diseases	
					Vaccines	

In Asia initiatives, we will aim for rapid approval with consideration for the use of overseas data.

Strategic Priority 4  
Reforming Operational Productivity



Making progress according to plan, targeting reductions of ¥10 billion in fiscal 2017



\* Benchmark: fiscal 2015

## SG&A expenses

- Optimizing workforce through early retirement
- Reducing companywide fixed expenses
- Focusing management resources
  - Transfer of generics business
  - End of Bipha's business
- Targeting a 5,000-person domestic workforce, moving ahead with reduction of work and reevaluation of duplicated functions

## Cost of sales

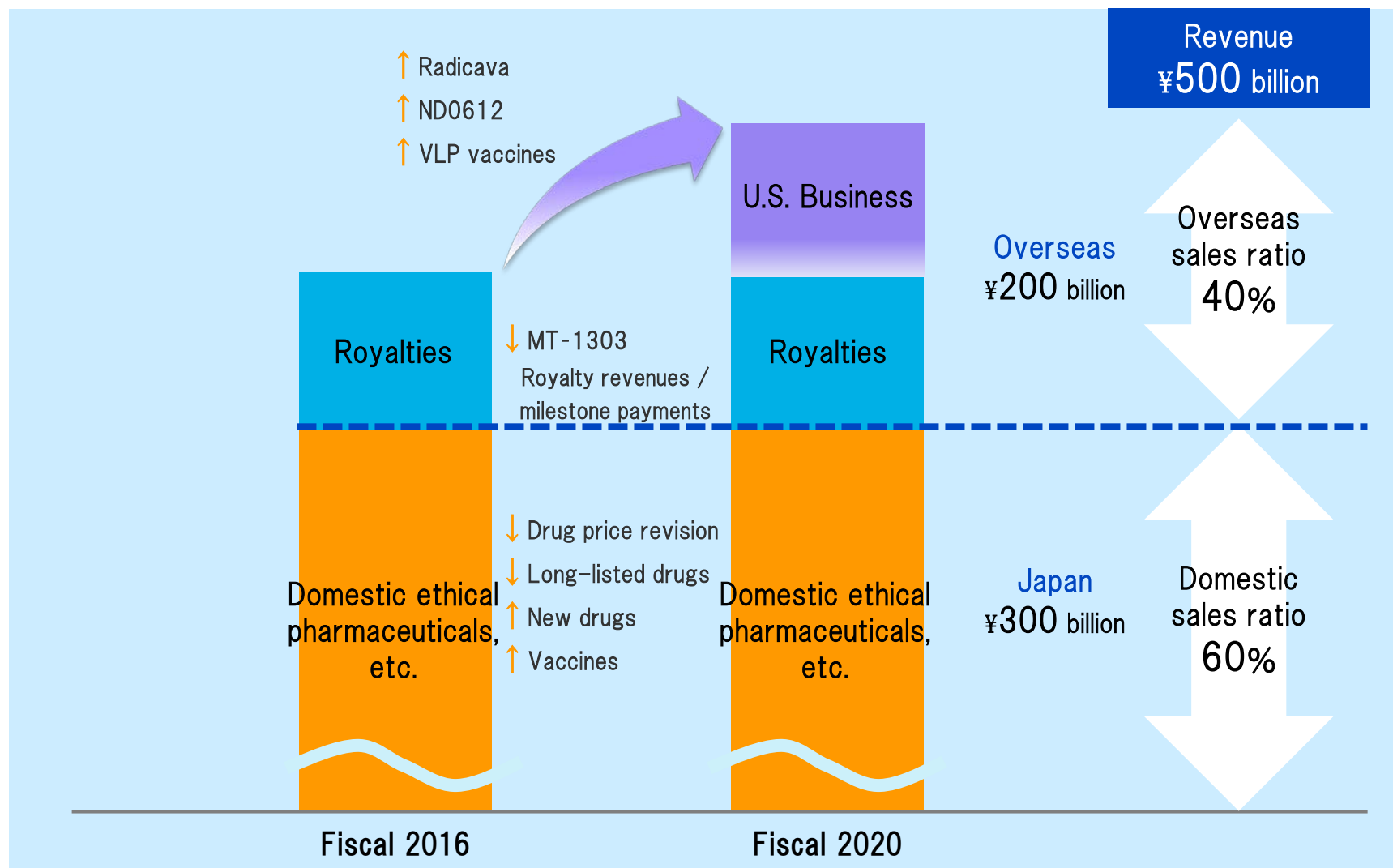
- Reevaluating procurement methods for pharmaceutical ingredients
- Increasing productivity by improving production technologies
- Optimizing production
- Optimizing distribution consignment system

Reinvestment in growth strategies

## Targeting the Objectives of the Medium-Term Management Plan16–20

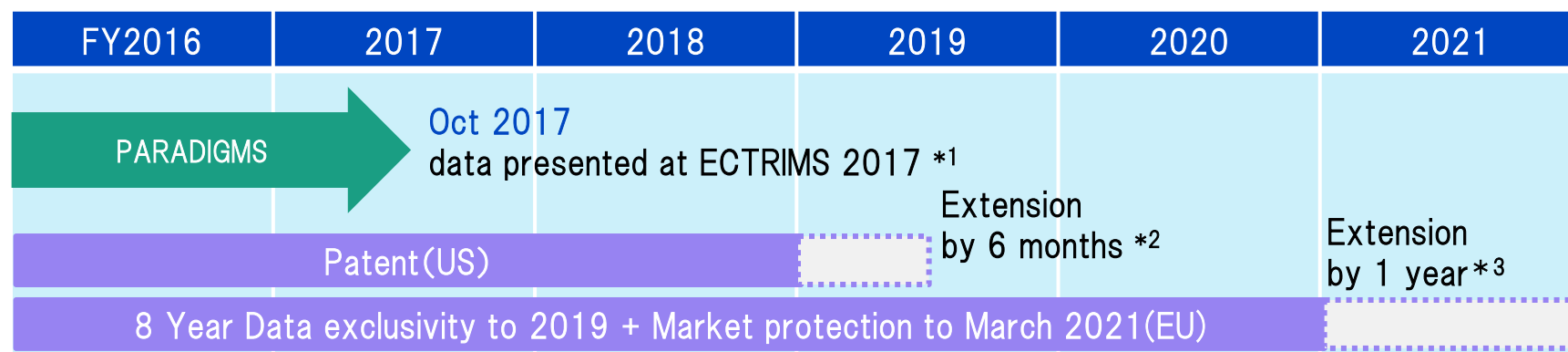


# Revenue



# Targeting the Objectives of the Medium-Term Management Plan16-20

## Gilenya (Novartis)

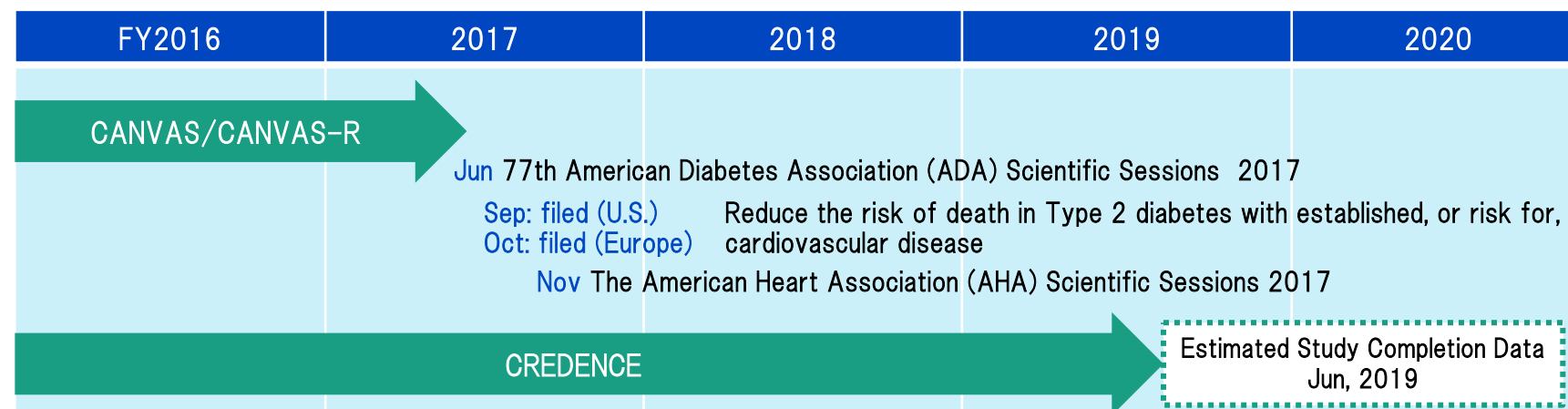


\*1) 82% lower relapse rate compared with interferon  $\beta$ -1a in pediatric patients with MS

\*2) 6 months US pediatric exclusivity granted, if requirements of the Written Request are met

\*3) If the pediatric indication is approved, possible extension of market protection by 1 year in EU to March 2022

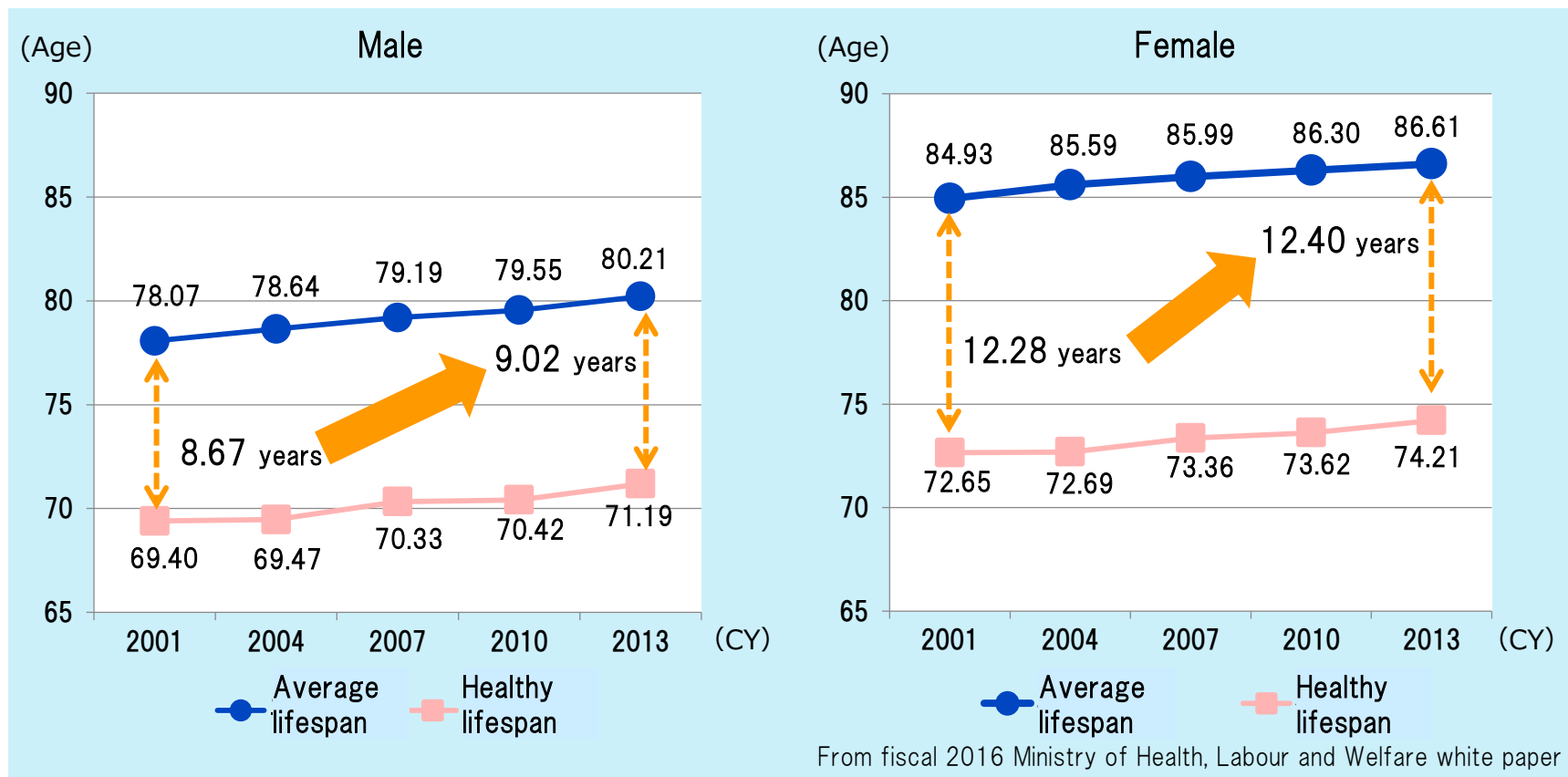
## INVOKANA(Janssen)



## Initiatives to Increase Corporate Value

The Social Value that We Are Aiming to Create

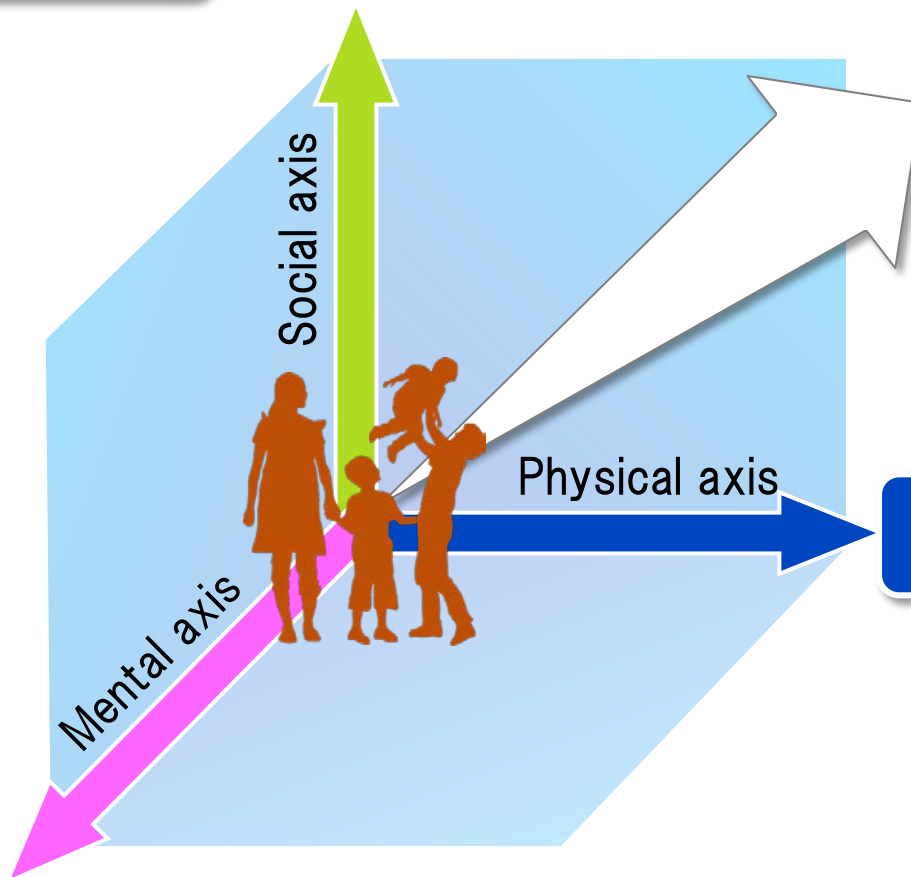
# The Mission of Pharmaceuticals: Difference Between Average Lifespan and Healthy Lifespan



- ▶ The difference between the average lifespan and health lifespan is one factor in the increases in health care expenditures and other social expenditures.
- ▶ Moving forward, extending the healthy lifespan and reducing this difference by as much as possible will be part of the mission of pharmaceuticals and of society as a whole.

Social  
well -being

**Activate Social Activities**  
(Better relationship with society)



Mental  
well -being

**Sense of Life-Fulfillment**  
(Sense of Peace of mind)

Physical  
well -being

**Extending Healthy Life**  
(Prevention / Disease control / Healing)

## KAITEKI LIFE

- ✓ Energetic Lifestyles
- ✓ Meaningful Lifestyles
- ✓ Sense of Contributing to Society

## Physical

## Contributing to healthy lifespan extension

Vision for  
the future

No restrictions in daily life due to health problems and able to live energetic lifestyles.

## Developing treatment agents

### Autoimmune diseases

ヒト型抗ヒトTNF $\alpha$ モノクローナル抗体製剤 [薬価基準収載]

**シンポニー®** 皮下注 50mg シリンジ

ゴリムマブ(遺伝子組換え)製剤  
Simponi® Subcutaneous Injection

生物由来製品 創薬 処方箋医薬品  
\*注意—医師等の処方箋により使用すること

抗ヒトTNF $\alpha$ モノクローナル抗体製剤 [薬価基準収載]

**レミケード®** 点滴静注用100

REMICADE® for I.V. Infusion 100 (インフリキシマブ(遺伝子組換え)製剤)

生物由来製品 創薬 処方箋医薬品 (注意—医師等の処方箋により使用すること)

Preventing joint  
destruction, restoring  
daily life

### Diabetes and kidney diseases

SGLT2阻害剤—2型糖尿病治療剤— [薬価基準収載]

**カナグル®錠100mg**

CANAGLU® Tablets 100mg (カナグリフロジン水和物錠)

処方箋医薬品 (注意—医師等の処方箋により使用すること)

選択的DPP-4阻害剤—2型糖尿病治療剤— [薬価基準収載]

**テネリア®錠20mg**

TENELIA® Tablets 20mg (チナリグリブチン臭化水素酸塩水和物錠)

処方箋医薬品 (注意—医師等の処方箋により使用すること)

**カナリア®配合錠**

CANALIA® COMBINATION TABLETS  
(チナリグリブチン臭化水素酸塩水和物/カナグリフロジン水和物配合錠)

処方箋医薬品 (注意—医師等の処方箋により使用すること) [薬価基準収載]

Limiting complications  
and transition to  
dialysis

### Central nervous system diseases

フリーラジカルスカベンジャー [薬価基準収載]

**ラジカット®** 注30mg  
点滴静注バッグ30mg

RADICUT® inj. 30mg (日本薬局方 エダラボン注射液)  
RADICUT® BAG for I.V. Infusion 30mg (日本薬局方 エダラボン注射剤)

処方箋医薬品 (注意—医師等の処方箋により使用すること)

**Radicava™**  
(edaravone) injection for infusion  
30 mg/100 mL

Limiting disease  
progress, enjoying  
daily life as long as  
possible

### Vaccines



Preventing spread of  
infectious diseases



# KAITEKI LIFE: Mental Axis and Social Axis Initiatives

## Mental

### Contributing to sense of self-fulfillment

Vision for  
the future

Feeling a sense of mental stability and fulfilling lifestyles

Building evidence for treatment  
agents (peace of mind in regard  
to medicines being used)



Enhancing accessibility to  
treatment



Increasing the health literacy  
of patients and families



Self medication  
(self management)

Compliance /  
lifestyle guidance  
application

## Society

### Contributing to dynamic social activities

Vision for  
the future

Social activities conducted without limitation, sense of  
contributing to society

Reducing treatment burden  
(social activities without limitation)



Social activities / employment  
(patient group support, work support, etc.)

Tenohira Partnership  
Program\*

\* System for providing financial assistance to associations  
and support groups for patients with incurable diseases

## Open Up the Future with Our Hands



### Cautionary Statement

The statements contained in this presentation is based on a number of assumptions and belief in light of the information currently available to management of the company and is subject to significant risks and uncertainties.