

Financial Results

FY2017

(January to December 2017)

Carna Biosciences, Inc.



Stock Code : 4572

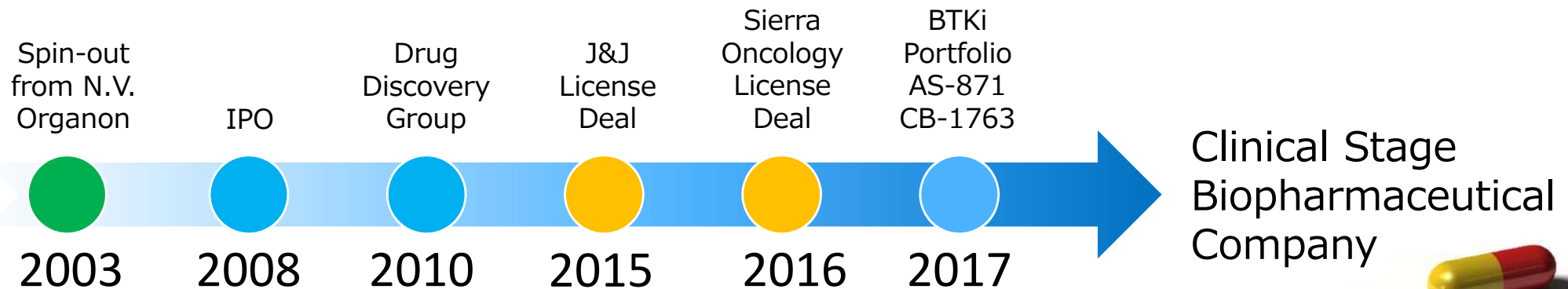
Building a Sustainable Company



Continuously Discovering and Delivering Innovative Therapies for Patients by leveraging Carna's powerful kinase technology platform

Creating Drug Discovery Pipeline

Continuing to Expand Kinase technology platform



Clinical Stage
Biopharmaceutical
Company



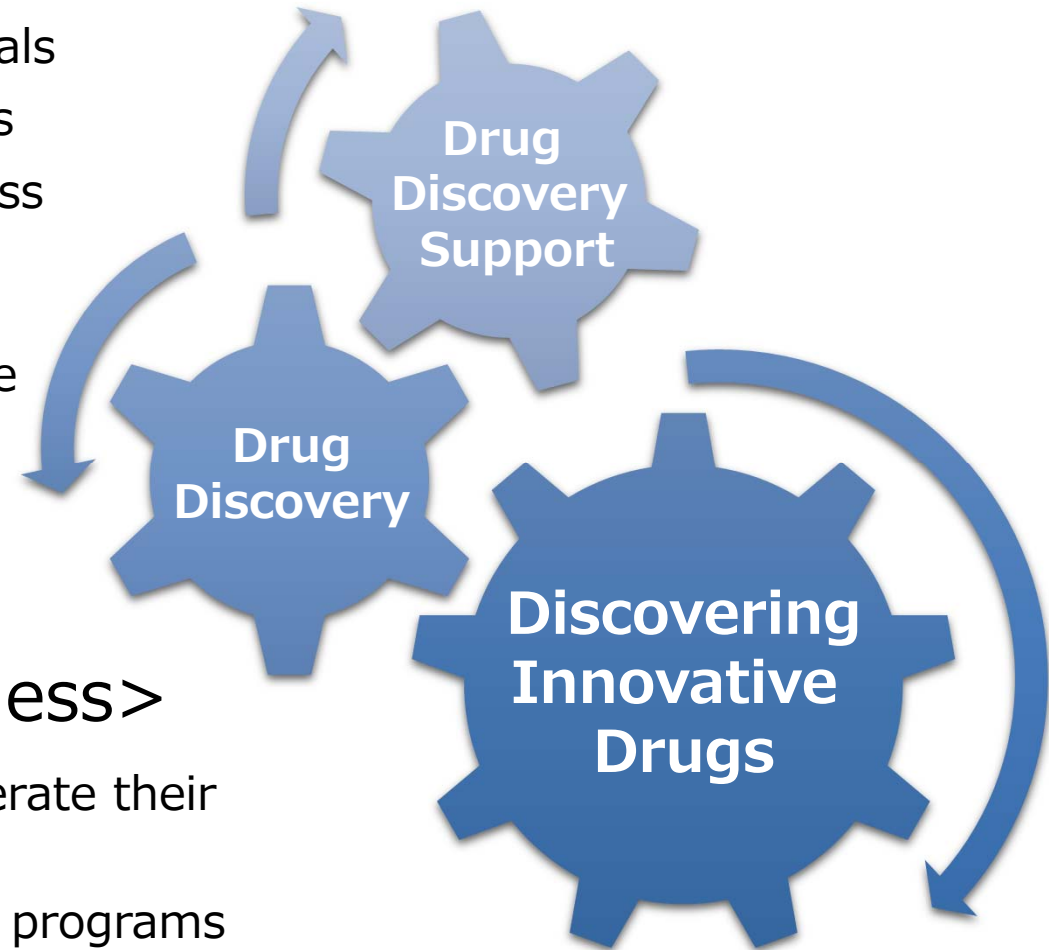
- ◆ Continuing to Expand Preclinical/Clinical Pipeline
- ◆ Building clinical development capabilities

<Drug Discovery>

- Establish elite team with talented professionals
- Focus on oncology and autoimmune diseases
- Well-balanced pipeline consists of first-in-class and best-in-class programs
- Building clinical development capabilities to demonstrate clinical activity of our innovative therapy

<Drug Discovery Support Business>

- Support pharmaceutical companies to accelerate their kinase inhibitor drug discovery
- Secure funds to invest in our drug discovery programs



- Two potential blockbusters, reversible BTK inhibitors, advanced into preclinical development
 - ✓ AS-871 (Autoimmune diseases)
 - ✓ CB-1763 (Cancer)
- Preclinical development of SRA141, a CDC7 inhibitor program out-licensed to Sierra Oncology, was advancing without issues.
- We strategically focused our marketing efforts on expanding sales of DGK assay kits. Some of our customers are evaluating the kits and we continue to make marketing efforts to win large-scale contracts.
- We issued series 16th and 17th subscription rights to shares to fund future R&D expenses, aiming to accelerate research and development further.

■ Pipeline Progress

- ✓ AS-871, a novel non-covalent BTK inhibitor targeting autoimmune diseases, advanced to preclinical stage in 2Q.
- ✓ CB-1763, a next generation BTK inhibitor targeting blood cancer, advanced to preclinical stage in 4Q.

■ Protecting intellectual properties globally

- ✓ CDC7 inhibitor: Patent registration in Japan, Australia, and Mexico
- ✓ TNIK inhibitor: Patent registration in the U.S., Japan, and in China
- ✓ BTK inhibitor: Patent registration in the U.S., Japan, Korea, and Australia

■ Collaborative Research Programs

- ✓ A new research collaboration with EpiBiome to develop potential new treatments in the area of microbiome in 1Q.
- ✓ Extended the research collaboration with the National Cancer Research Institute to develop novel anticancer drugs targeting kinases in 1Q.
- ✓ Renewed the research collaboration with Hiroshima University to discover novel drugs targeting leukemia stem cells in 2Q.
- ✓ A new research collaboration with Keio University School of Medicine to establish a new cancer immunotherapy in 2Q.
- ✓ The research collaboration with Osaka Prefecture University was extended in 3Q to develop novel strategies for structure-based drug design.
- ✓ Continuing the research collaboration with Ehime University School of Medicine to identify a new drug target in 4Q.

Pipeline Status



Compound	Target Kinase	Indication	Development Phase						
			Lead generation	Lead optimization	Candidate selection	Preclinical trials	PhI	PhII	PhIII
SRA141 (AS-141)	CDC7	Cancer							
NCB-0846	Wnt-signal (TNIK)	Cancer							
AS-871	BTK	Autoimmune Diseases			→ advanced				
CB-1763	BTK	Blood Cancer Immuno-Oncology			→ advanced				
NCB-0594	Wnt-signal (TNIK)	Cancer Immuno-Oncology							
Small Molecule	TGFβ signaling	Blood Cancer Immuno-Oncology							
Small Molecule	Kinase	Autoimmune Diseases	→ advanced						
Small Molecule	N/A	Malaria							
Small Molecule	Kinase	Neurodegenerative disease							
Small Molecule	DGK	Immuno-Oncology	new						

- Immunotherapeutic interventions, including immune checkpoint inhibitors, have emerged as a new promising therapeutic approach for a wide range of cancer types.
- However, only a small subset of cancer patients respond to immunotherapy, such as anti-PD1 antibody.
- Therefore, development of a novel therapeutic approach targeting immune checkpoints is highly demanded.

Novel kinase targets modulating immune checkpoints

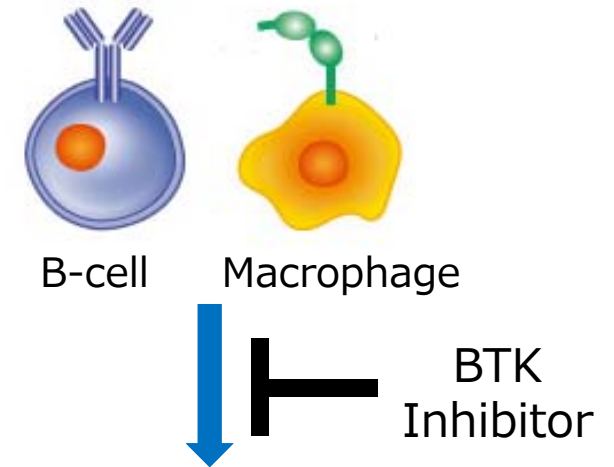
Carna's powerful kinase
drug discovery engine



Initiate next generation research programs

Bruton's Tyrosine Kinase (BTK)

- ✓ BTK is one of the crucial kinases for the B-cell maturation and macrophage activation
- ✓ BTK has been recognized as a validated therapeutic target since the success of Ibrutinib, the first FDA approved BTK inhibitor
- ✓ The expected peak sales of Ibrutinib is > US\$7bn*



◆ BTK inhibitors are high value assets

Year	Company	Target	Total Value
2011	J&J / Pharmacyclics	Cancer	\$975m
2015	Eli Lilly / Hanmi	Autoimmune	\$690m
2015	AstraZeneca / Acerta	Cancer	\$7bn (M&A)
2015	AbbVie / Pharmacyclics	Cancer	\$21bn (M&A)
2017	Sanofi / Principia	Autoimmune	\$800m
2018	TG Therapeutics/ Jiangsu Hengrui Medicine	Cancer	\$350m

Blood Cancer

e.g. B-cell malignancies

Autoimmune diseases

e.g. Rheumatoid arthritis, asthma, systemic lupus erythematosus

Portfolio of Two BTK Inhibitors



- ◆ Most of BTK inhibitors in clinical development are covalent/irreversible inhibitors
- ◆ Carna is developing next generation BTK inhibitors with unique properties

AS-871	CB-1763
<ul style="list-style-type: none">• highly selective• non-covalent/reversible inhibitors• significant efficacy in arthritis model <p>Development undergoing targeting <u>autoimmune diseases</u></p>	<ul style="list-style-type: none">• highly selective• non-covalent/reversible inhibitors• inhibits both BTK wild type and BTK C481 mutants• strong anti-tumor effects in lymphoma model <p>Development undergoing targeting <u>cancer</u></p>

AS-871

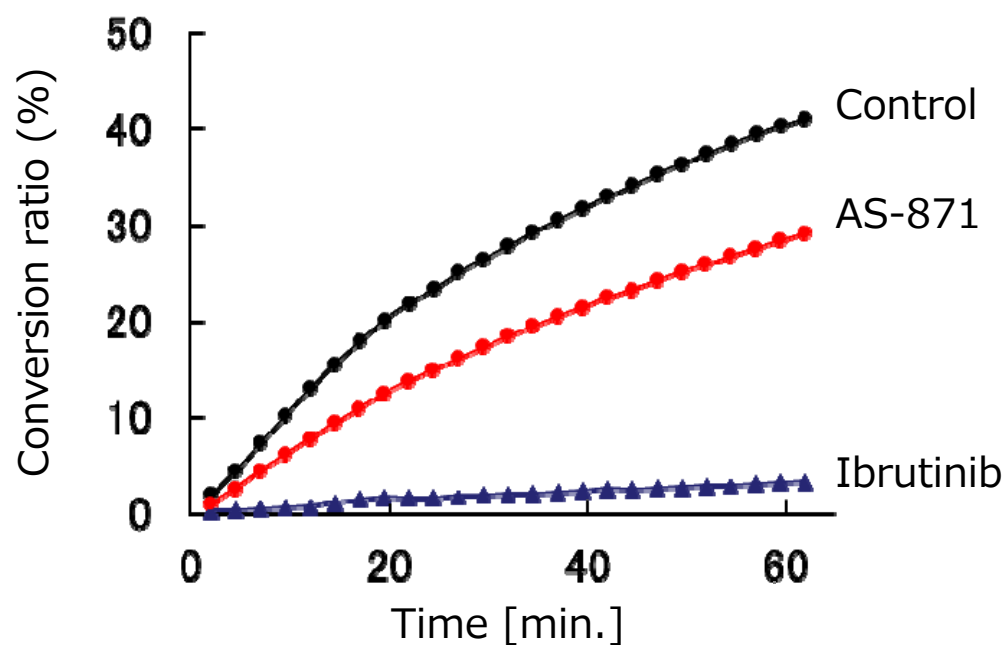
Development undergoing
targeting autoimmune diseases

- Small molecule BTK inhibitor
- Non-covalent/reversible
- High kinase selectivity
- Demonstrated significant efficacies in arthritis models
- Showed efficacy in systemic lupus erythematosus model
- Preclinical development undergoing with IND submission targeted in the first half of 2019

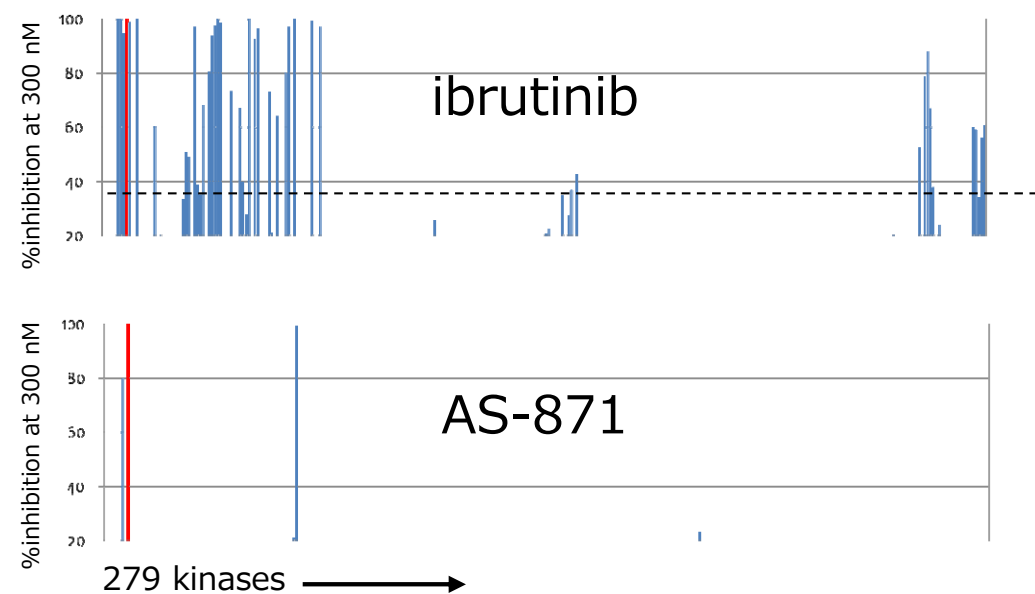
- ✓ Non-covalent/Reversible inhibition
- ✓ High selectivity

 develop targeting autoimmune diseases

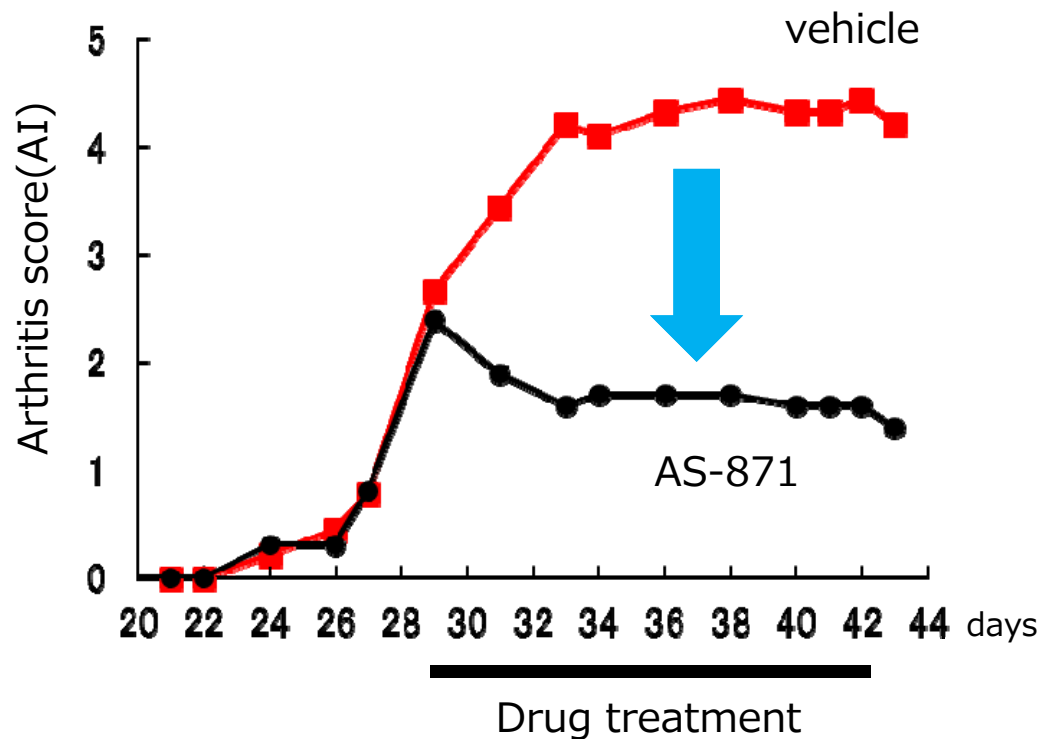
◆ Non-covalent/Reversible inhibition



◆ High kinase selectivity

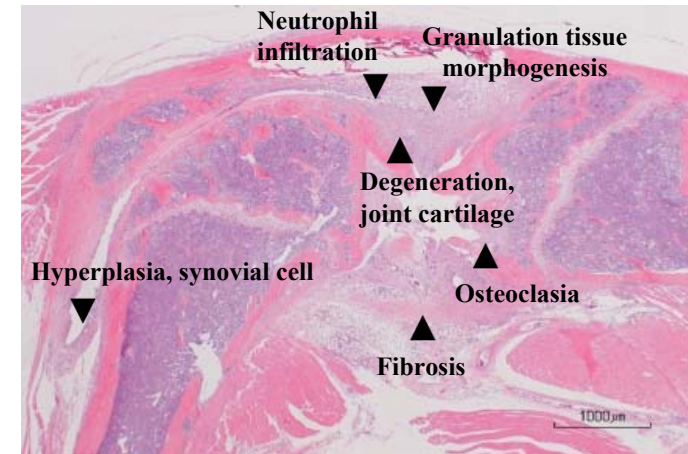


Therapeutic efficacy in Collagen-induced arthritis (CIA) mice

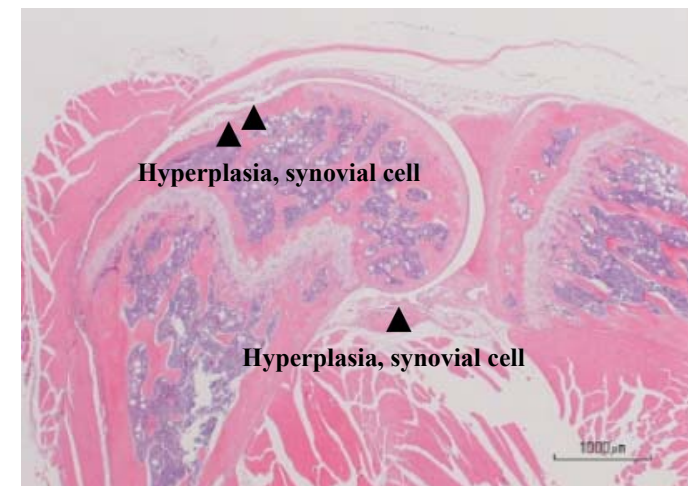


Histopathology of knee joints

vehicle



AS-871



CB-1763

Development undergoing
targeting cancer

- Small molecule BTK inhibitor
- Non-covalent/reversible
- High kinase selectivity
- Inhibits both BTK wild type and ibrutinib resistant BTK C481 mutants
- Displayed strong anti-tumor effects in lymphoma model
- Preclinical development undergoing with IND submission targeted in the first half of 2019
- Potential applications for autoimmune diseases

CB-1763: Next Generation BTK Inhibitor



- Recent studies indicated that emergence of BTK mutations cause ibrutinib resistance
- A selective and non-covalent BTK inhibitor is highly demanded to overcome ibrutinib resistance, emerging unmet medical need

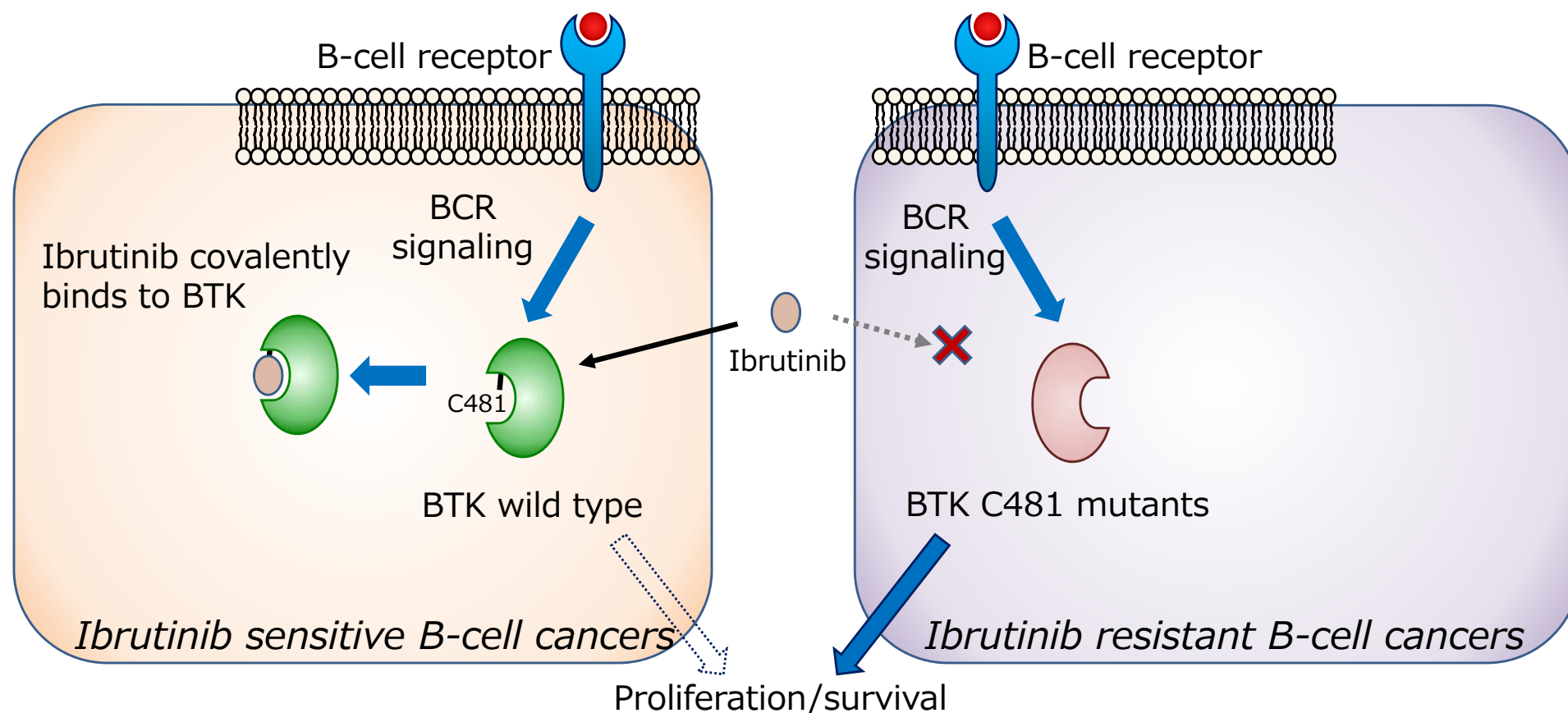
VOLUME 35 • NUMBER 13 • MAY 1, 2017

JOURNAL OF CLINICAL ONCOLOGY

ORIGINAL REPORT

BTK^{C481S}-Mediated Resistance to Ibrutinib in Chronic Lymphocytic Leukemia

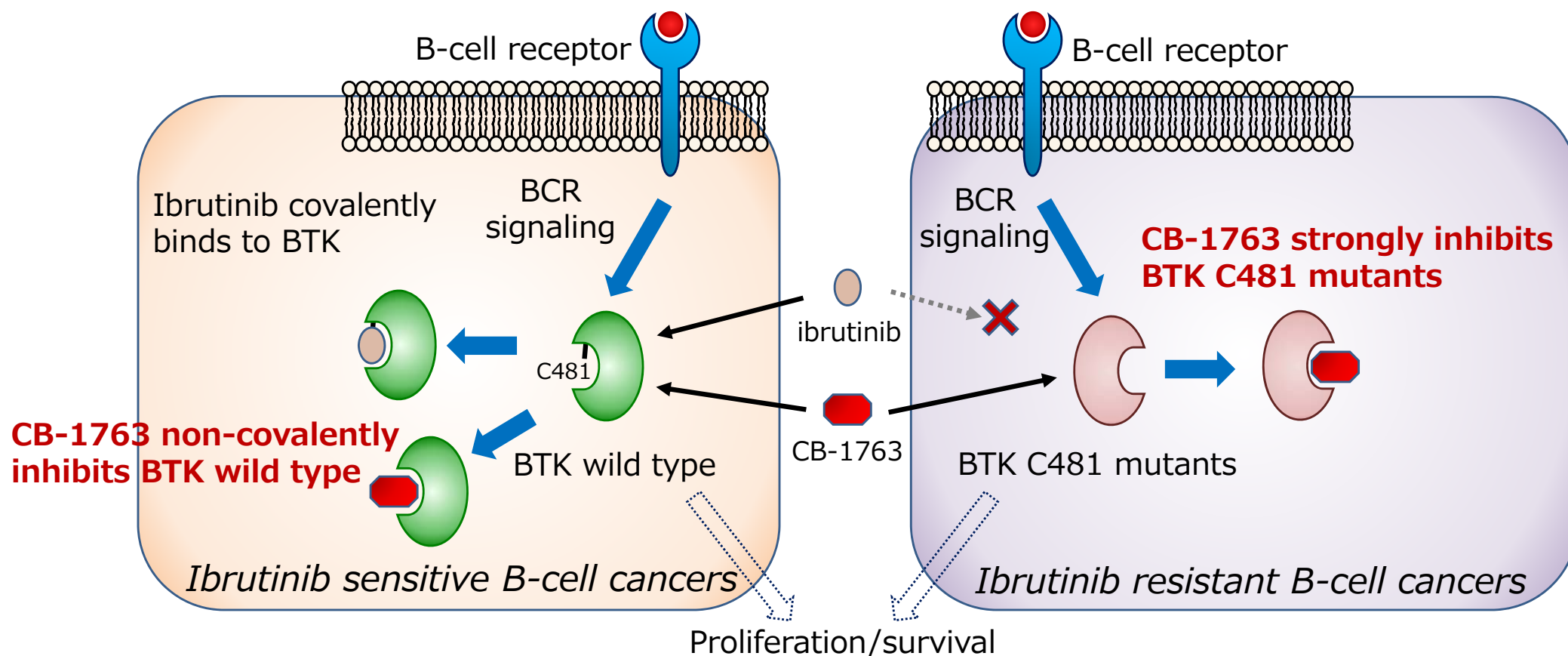
Jennifer A. Woyach, Amy S. Ruppert, Daphne Guinn, Amy Lehman, James S. Blachly, Arletta Lozanski, Nyla A. Heerema, Weiqiang Zhao, Joshua Coleman, Daniel Jones, Lynne Abruzzo, Amber Gordon, Rose Mantel, Lisa L. Smith, Samantha McWhorter, Melanie Davis, Tzyy-Jye Doong, Fan Ny, Margaret Lucas, Weihong Chase, Jeffrey A. Jones, Joseph M. Flynn, Kami Maddocks, Kerry Rogers, Samantha Jaglowski, Leslie A. Andritsos, Farrukh T. Awan, Kristie A. Blum, Michael R. Grever, Gerard Lozanski, Amy J. Johnson, and John C. Byrd



CB-1763: Next Generation BTK Inhibitor



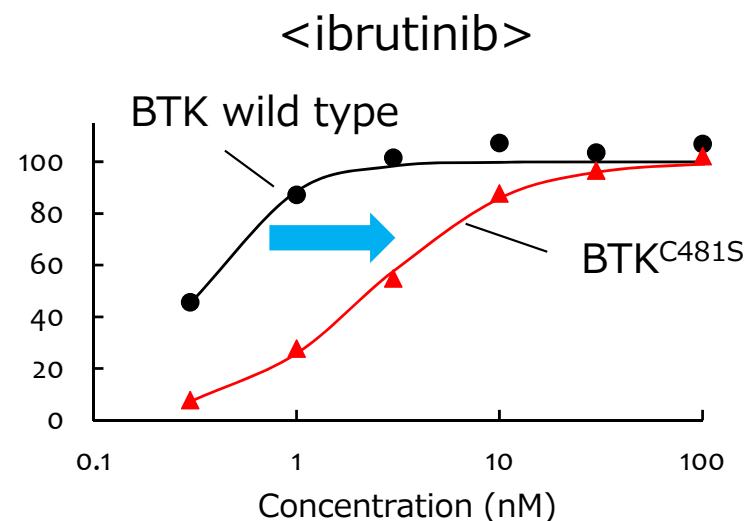
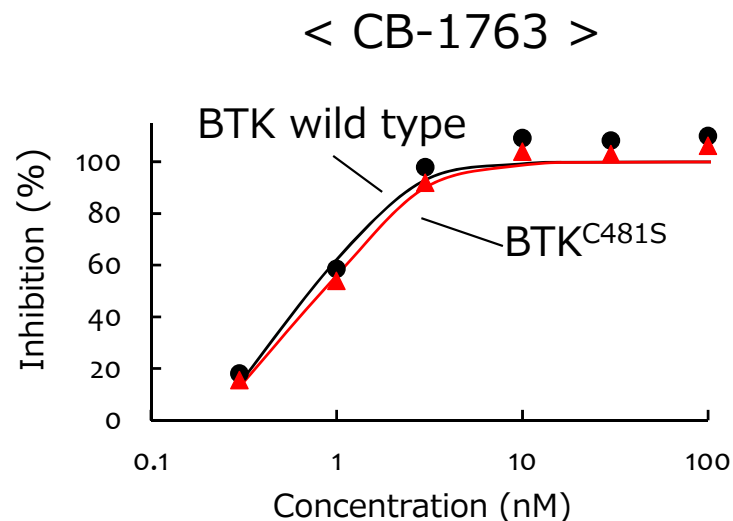
CB-1763 is a next generation non-covalent BTK inhibitor, designed to inhibit both BTK wild type and BTK C481 mutants in a highly selective and reversible manner.



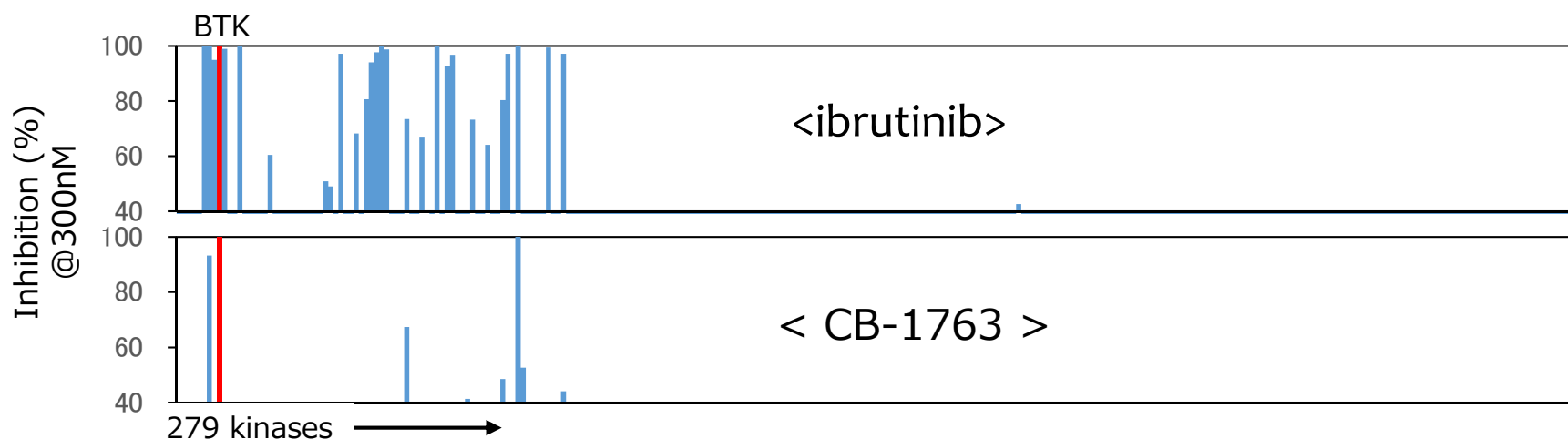
CB-1763 retains potency for BTK mutant



- CB1763 inhibits both WT and C481S mutant BTK enzymes



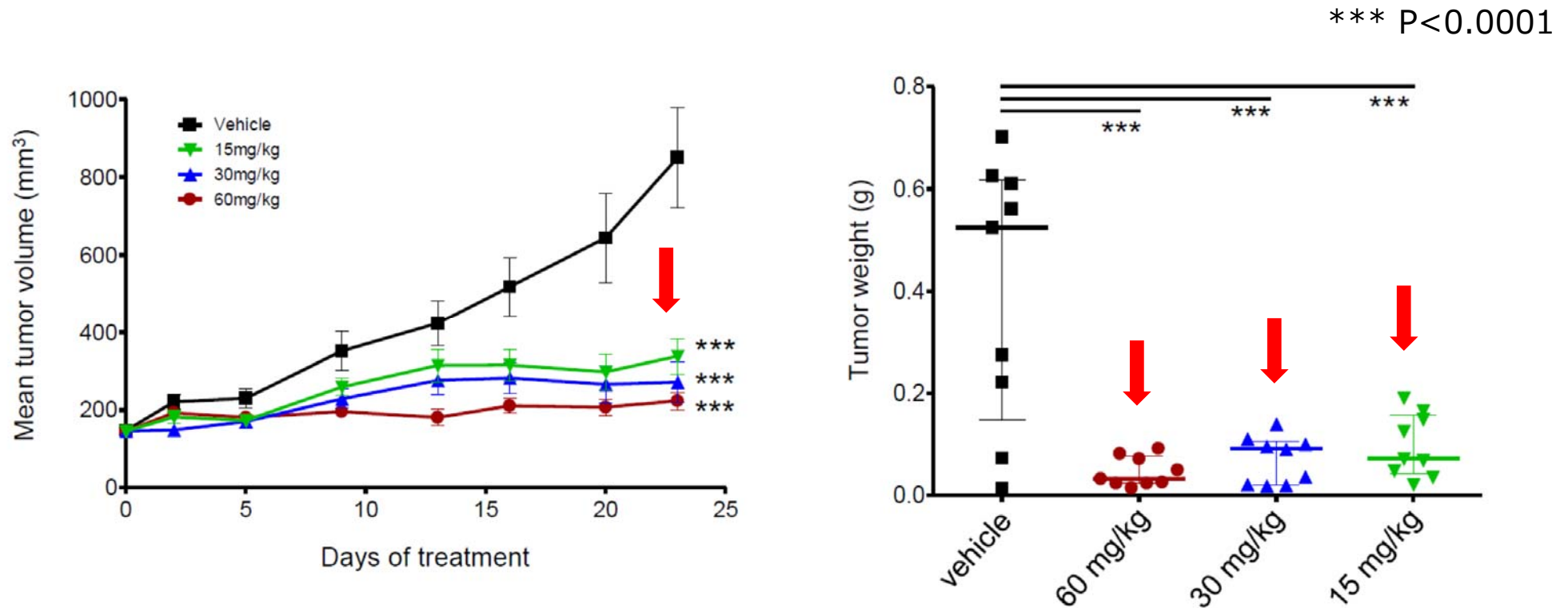
- CB-1763 is a highly selective inhibitor



CB-1763 showed strong anti-tumor effects



Mice bearing OCI-LY10 xenograft tumors were dosed orally with CB-1763 twice a day



CB-1763 significantly inhibits tumor growth in a B-cell lymphoma mouse model

Summary of Drug Discovery Support Business



- ✓ In Japan, sales to major customers were steady except Ono Pharmaceutical.
- ✓ In North America, increased sales driven by cell-based assay services and others.
- ✓ In Asia, strong growth in China and Korea. Expand business in these countries further.
- ✓ In Europe, strong sales growth of kinase proteins and profiling services covered decreases in cell-based assay services.
- ✓ Successful launch of our proprietary DGK assay kits. We continue to focus on acquiring large-scale contracts with global pharmas for supplying DGK assay kits.

- Expansion of our products and service business portfolio to support customers' research
- ✓ Step into emerging science: Microbiome. Carna started to provide microbiome profiling services in Japan by collaborating with EpiBiome, a precision microbiome engineering company in 3Q.
- ✓ Delivering new technology: fluorescence-based kinase assay kits by collaborating with AssayQuant technologies, Inc. in 4Q.

FY2017

Consolidated Financial Results



(JPY mn)	FY2016 Actual	FY2017 Actual	YoY Change	FY2017 Revised plan	Vs. Revised Plan	
Sales	811	657	-154 -19.0%	701	93.7%	Recorded initial upfront payment of 98 mn yen in FY2016.
Operating Loss	(423)	(699)	-275	(727)	—	Increase in R&D cost Recorded initial upfront payment of 98 mn yen in FY2016.
Ordinary Loss	(440)	(711)	-270	(738)	—	
Net Loss	(289)	(737)	-447	(766)	—	Recorded income from sales of investment securities in FY2016.
R&D Cost	513	670	+157 +30.7%			Investment in preclinical study of pipeline

Note 1: Rounded down to the nearest million yen.

Note 2: YoY change % and comparison to FY2017 plan for Operating Loss, Ordinary Loss, and Net Loss are not presented since losses were recorded.

Note 3: Revised FY2017 plan was disclosed on November 10, 2017.

FY2017

Results by Business Segment



(JPY mn)	FY2016 Actual	FY2017 Actual	YoY Change	FY2017 Revised Plan	Vs. Revised Plan	
Total Sales	811	657	-154 -19.0%	701	93.7%	
Drug Discovery Support	712	657	-55 -7.7%	701	93.7%	Increase in U.S. sales Decrease in sales to Ono Pharmaceutical
Drug Discovery & Development	98	—	-98	—	—	Recorded initial upfront payment of 98 mn yen in FY2016.
Total Operating Loss	(423)	(699)	-275	(727)	—	
Drug Discovery Support	192	142	-49 -25.6%	173	82.1%	Decrease in sales
Drug Discovery & Development	(616)	(841)	-225	(901)	—	Investment in preclinical study expenses Recorded initial upfront payment of 98 mn yen in FY2016.

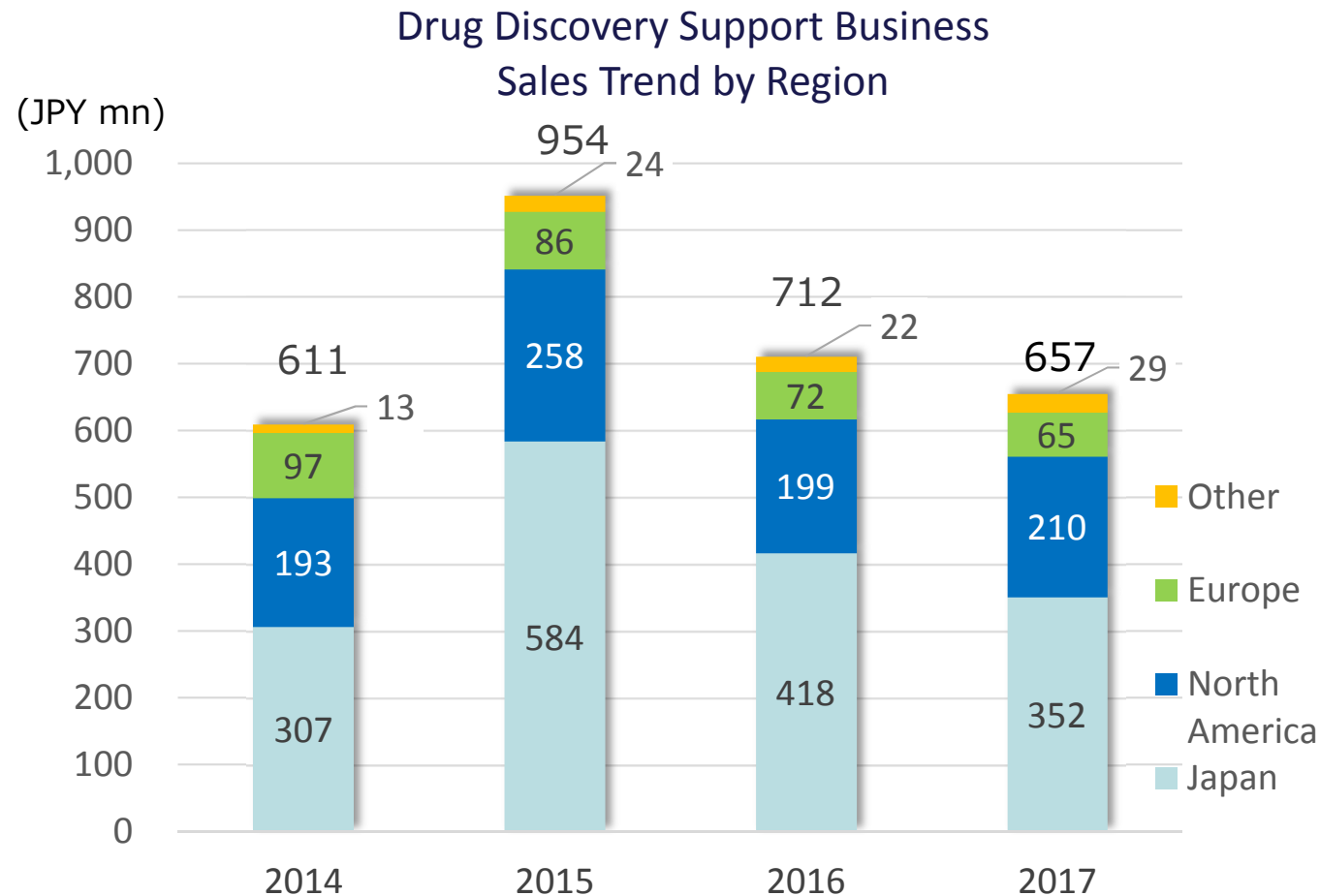
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Note 3: Revised FY2017 plan was disclosed on November 10, 2017.

FY2017 Sales Trend by Region

Drug Discovery Support Business



- Japan: Decreased 15.8% YoY
Sales to other companies were firm while sales to Ono Pharmaceutical were weak.
- North America: Increased 5.4% YoY
Cell-based assay services were robust
- Europe: Decreased 9.2% YoY
A decrease in cell-based assay services was offset by robust kinase proteins sales and profiling service
- Other: Increased 31.7%YoY
Strong increase in China and Korea

(Note) Sales to Ono Pharmaceuticals
FY2016 ... 194 mn yen
FY2017 ... 144 mn yen

Consolidated Balance Sheet



(JPY mn)

	As of Dec. 31, 2016	As of Dec. 31, 2017	Change	Reason for changes
Current assets	2,492	2,134	-358	
Cash and deposits	2,161	1,856	-304	
Non-current Assets	73	56	-17	
Total assets	2,566	2,190	-375	
Current liabilities	271	341	+ 70	Current portion of long-term loans payable +11, Account payable-other + 47
Non-current liabilities	555	470	-85	Long term loans payable -57, Bonds payable -28
Total liabilities	826	812	-14	
Total net assets	1,739	1,377	-361	Capital stock and capital surplus + 367, Retained earnings-737
Total liabilities and net assets	2,566	2,190	-375	

Shareholders' equity ratio	67.6%	62.2%
BPS	187.73 yen	142.68 yen
PBR	11.32 x	7.91 x
Share price of Carna Biosciences	2,125 yen	1,128 yen

Note: Share price of Carna Biosciences is based on the closing price of JASDAQ growth

■ Series 16th and 17th Subscription Rights to Shares

Series	Status of Exercise (as of end of January 2018)
Series 16th Subscription Rights to Shares	Total Number of shares exercised 226,000 shares (24.30% of total rights) Total value exercised 287,010,000 yen
Series 17th Subscription Rights to Shares	Not exercised

■ Bank Loan

(1) Lender	The San-in Godo Bank
(2) Total Amount	300 million yen
(3) Interest Rate	1.70% per annum (fixed rate)
(4) Date of loan	January 26, 2018
(5) Term	3 years

Business Plan for FY2018

<Drug Discovery>

- SAR141: Milestone payment upon initiation of Phase I study (US\$ 4million)
- AS-871: Initiate GLP toxicity studies
- CB-1763: Formulation, establish manufacturing process, and kg-scale production

<Drug Discovery Support>

- Achieve sales target of 750 million yen.
- Add further revenue to the earnings plan by strategic focus on expanding DGK and other lipid kinases.
- Boost sales in North America.

FY2018 Financial Plan



(JPY mn)	FY2017 Actual	FY2018 Plan	YoY Change	
Total Sales	657	1,190	+533 +81.1%	
Drug Discovery Support	657	750	+93 +14.2%	Expand sales in the U.S.
Drug Discovery & Development	—	440	+440	Milestone payment from Sierra upon initiation of Phase I clinical study
Total Operating Loss	(699)	(679)	+19	
Drug Discovery Support	142	150	+7 +5.2%	
Drug Discovery & Development	(841)	(829)	+12	Milestone payment from Sierra Invest in R&D
Ordinary Loss	(711)	(694)	+17	
Not Loss	(737)	(758)	-20	Impairment loss due to capex
R&D Cost	670	1,014	+343	Investment in preclinical study to advance AS-871 and CB1763
Capex	18	63	+45	Equipment for R&D

Note 1: Rounded down to the nearest million yen.

Note 2: YoY change % and comparison to FY2017 plan for Operating Loss, Ordinary Loss, and Net Loss are not presented since losses were recorded.

Drug Discovery Support: Sales Plan

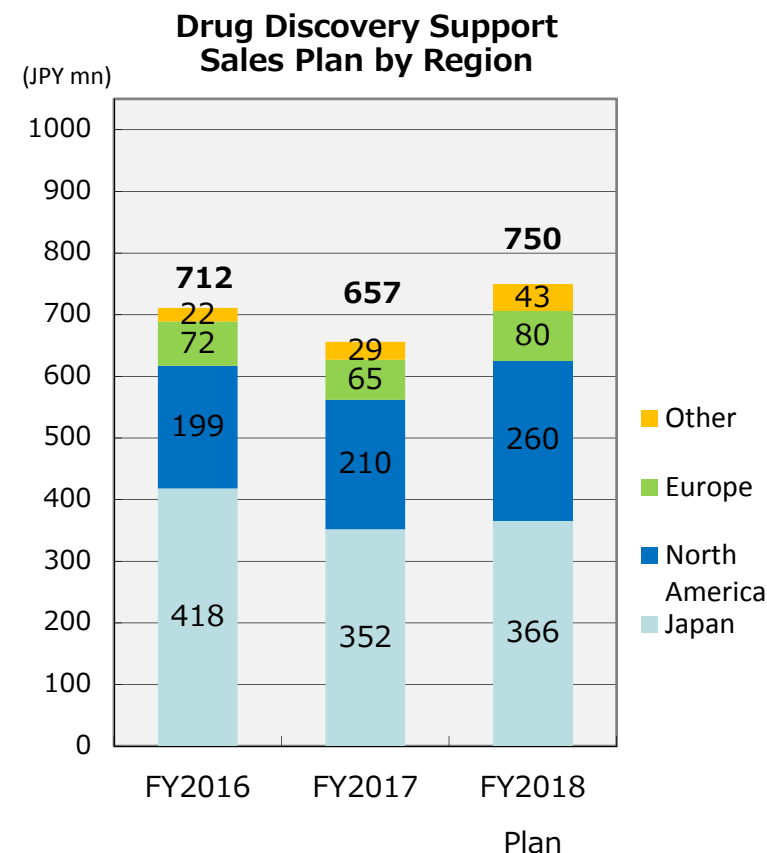


(JPY mn)	FY2016 Actual	FY2017 Actual(a)	FY2018 Plan (b)	Change (b)-(a)
Drug Discovery Support	712	657	750	+93
Protein Kinases	248	241	279	+38
Assay Development	49	35	57	+22
Profiling & Screening	276	257	269	+12
ProbeX	15	9	—	-9
Crystallography (agent business)	11	19	25	+6
Cell-based Assay Services (agent business)	106	90	119	+28
Others	4	4	—	-4

Exchange rate(US\$):	108.81 yen	112.17 yen	110 yen
% of Overseas sales:	41.3%	46.4%	51.2%

Note: Actual foreign exchange rate is average rate of the term

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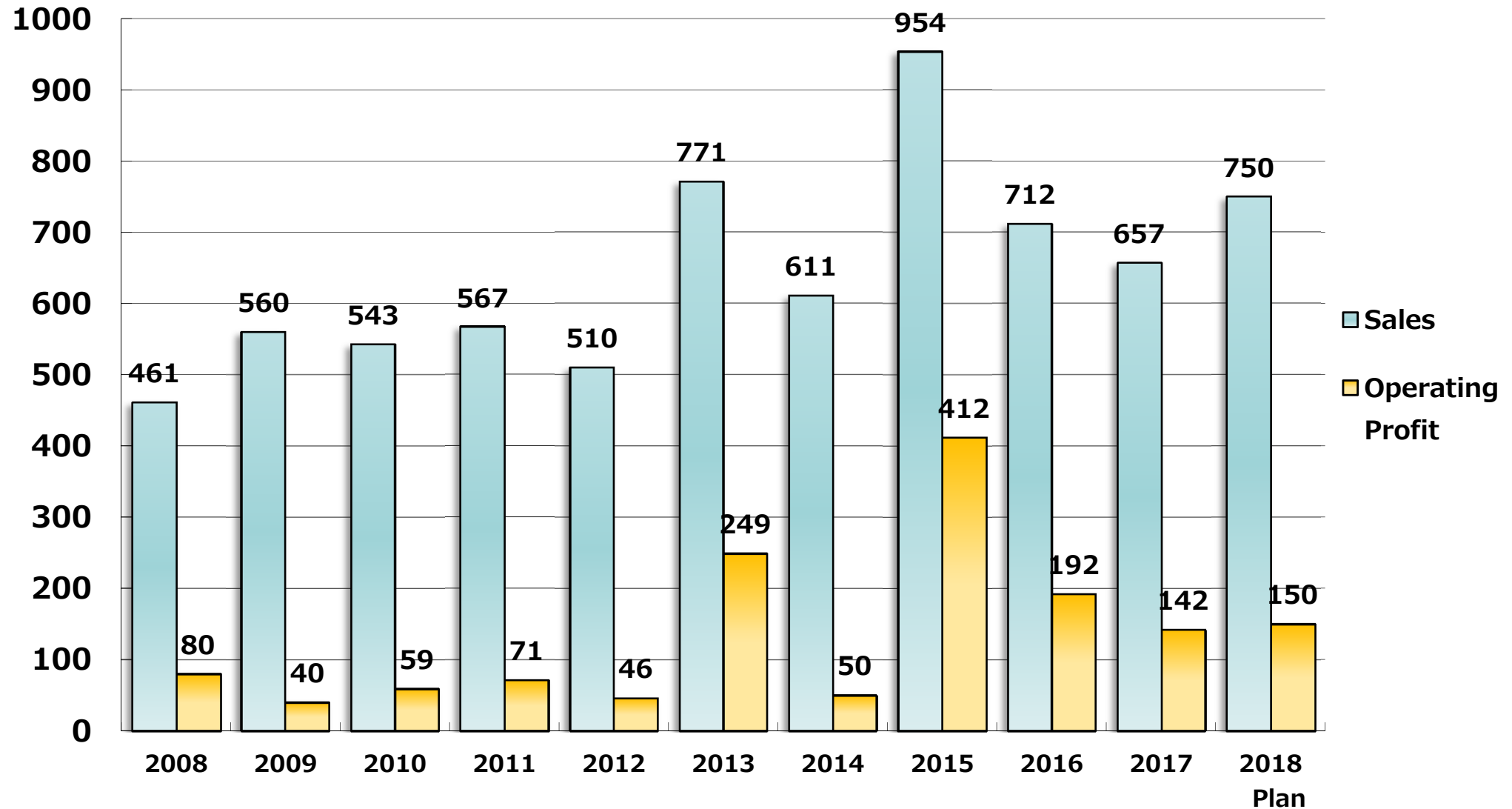


- ✓ Frequent customer visit to grow our customer base in North America
- ✓ Aim to beat the plan by acquiring large-scale contracts with global pharmas for supplying DGK assay kits

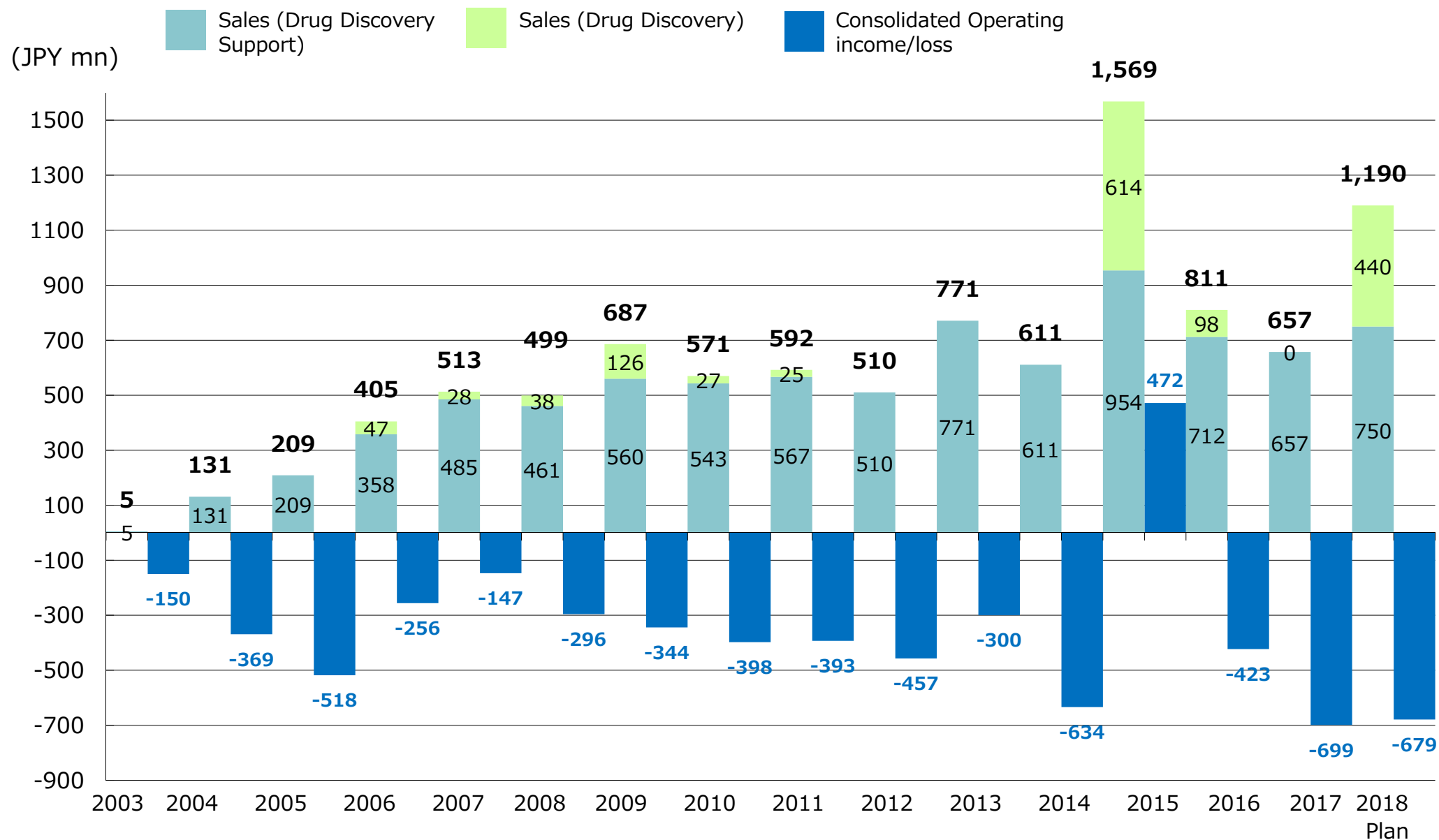
Drug Discovery Support: Sales and Operating Profit Plan

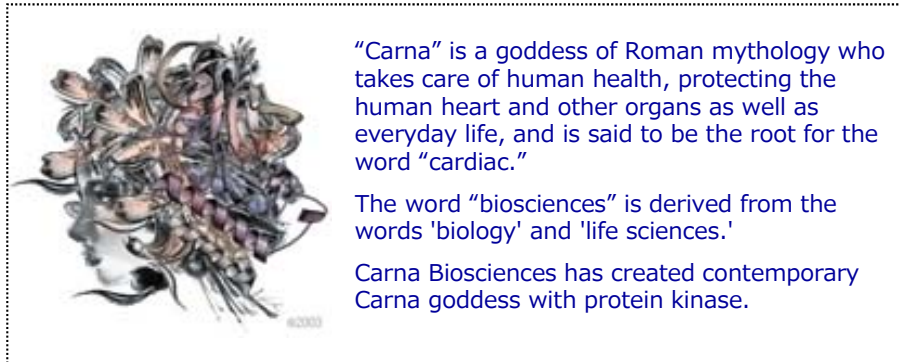


(JPY mn)



Consolidated Sales, Operating Profit, Net Profit





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Investors should aware that the actual performance of the company could be materially different from our current forecasts.

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