

Financial Results FY2019

(January to December 2019)

Carna Biosciences, Inc.



Stock Code: 4572

Strategic Objectives

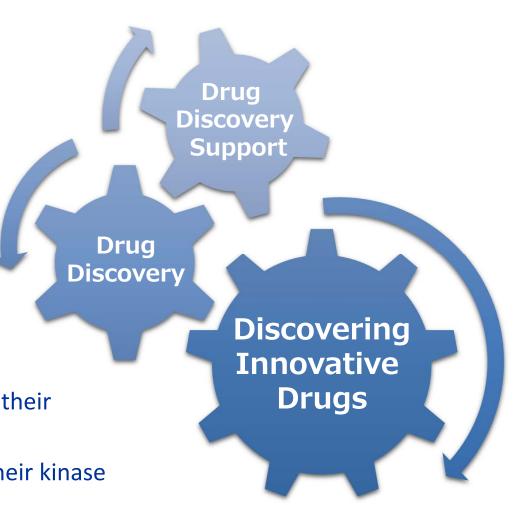


<Drug Discovery>

- 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
- Provide researchers with the new tools to drive their kinase research
- Secure funds to invest in our drug discovery programs



FY2019 Executive Summary



- Entered into a \$450 million license agreement with Gilead Sciences, Inc. to develop and commercialize small molecule compounds in immuno-oncology.
- Filed a Clinical Trial Application (CTA) in Netherlands for AS-0871, an investigational small molecule drug designed to non-covalently inhibit BTK.
- Recorded JPY1,000 million in sales at Drug Discovery Support business.

Progress and Milestones in 2019



<Drug Discovery>

- SRA141: Milestone payment upon initiation of Phase I study (\$4.0 million)
 - Sierra is conducting a campaign intended to seek non-dilutive strategic options to support further advancement of SRA141 to focus its resources on P3 study of momelotinib
- AS-0871: Submit a Clinical Trial Application (CTA) and Initiate Phase I study
 - → Submitted a CTA in December 2019
- AS-1763: Submit an Initial new Drug (IND) application
 - Advancing preclinical studies to submit an IND in 2020

<Drug Discovery Support>

- Achieve sales target of JPY800 million
 Achieved sales of JPY1,070 million
- Boost sales in North America
 Sales increased by 160.5% yoy in USD
- Establish new services including NanoBRET
 Steadily expanding



Drug Discovery and Development

Drug Discovery FY2019 Key Highlights



- Opened a clinical development office in South San Francisco to commence clinical studies of our drug candidates in the U.S. (February).
- Entered into a \$450 million license agreement with Gilead Sciences, Inc. to develop and commercialize small molecule compounds in immuno-oncology (June).
- Filed a Clinical Trial Application (CTA) for AS-0871 to initiate our first clinical trail in Europe (December).
- Fostering intellectual property activities on a global level
- ✓ BTK inhibitor: Patent registration in China
- ✓ Wnt signal inhibitor: Patent registration in Japan
- ✓ CDC7 inhibitor: Patent registration in Japan and Korea
- ✓ Antimalaria drug: Patent registration in the U.S.

Deal with Gilead for Immuno-Oncology



Carna grants Gilead worldwide exclusive rights for the development, commercializing inhibitors against an immuno-oncology target

Deal Size Upfront payment \$20 million

\$450 million in potential milestone payments upon achievement of certain

development and commercial milestones

Royalties on future net sales Royalties

Drug Discovery



* Carna also provides Gilead an exclusive access to Carna's proprietary lipid kinase drug discovery platform for value to support the development at Gilead.

Drug Discovery Support

Royalties

Robust Preclinical Pipeline



Compound	Target	Indication	Discovery	Preclinical	Clinical	Partner
SRA141 (AS-141)	CDC7/ASK	Cancer			IND Completed	SIERRA
Small Molecule	Kinase	Immuno-Oncology				GILEAD
Small Molecule	Kinase	Psychiatry & neurology				Sumitomo Dainippon Pharma
AS-0871	ВТК	Immune- inflammatory diseases			CTA Completed	
AS-1763	ВТК	Blood Cancer Immuno-Oncology				
Small Molecule	Wnt-signal	Immuno-Oncology				
Small Molecule	TGFβ signaling	Blood Cancer Immuno-Oncology				
Small Molecule	Kinase	Immune- inflammatory diseases				
Small Molecule	N/A	Malaria				
Small Molecule	Undisclosed	Cancer				

SRA141: CDC7 Inhibitor Targeting Cancer (Out-licensed to Sierra Oncology)



- ✓ Sierra has successfully completed the IND filing process with FDA, and plans to conduct a Phase 1/2 study in patients with colorectal cancer. Carna will receive the first milestone payment (\$4.0 million) upon the initiation of Phase 1 study.
- ✓ Sierra announced that it is conducting a campaign intended to seek non-dilutive strategic options to support further advancement of SRA141.
- ✓ Carna eligible for up to \$270 million in total milestone payments and royalties.

BTK Inhibitor Program

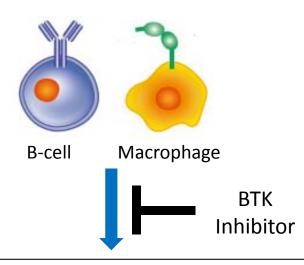


Bruton's Tyrosine Kinase (BTK)

- ✓ 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 > \$9 billion*



Year	Product	Company	Target	2018	2024 Est.
2013	Iburutinib	AbbVie/J&J	Blood cancer	\$4.4B	\$9.5B*1
2017	Acalabrutinib	Astra Zeneca	Blood cancer	\$62M*2	



Blood Cancer
e.g. B-cell malignancies
Autoimmune diseases
e.g. Rheumatoid arthritis, asthma, systemic lupus erythematosus

- In January 2019, Loxo Oncology, developing kinase inhibitors including non-covalent BTK inhibitor LOXO-305, was acquired by Eli Lilly for \$8.0 billion.
- In December 2019, ArQule, developing non-covalent BTK inihibotr ARQ 531, was acquired by Merck for \$2.7 billion.



High potential of non-covalent BTK inhibitors for sizable license deals

Source: 1. Evaluate Pharma

2. AstraZeneca Presentation

Non-covalent BTK Inhibitors Under Clinical Development by Competitors



◆ Under development targeting autoimmune diseases

Compound	Company	Development Phase
BMS-986142	BMS	P2
Fenebrutinib (GDC-0853)	Roche / Genentech	P2

◆ Under development targeting cancer

Compound	Company	Development Phase
vecabrutinib (SNS-062)	Sunesis	P1b/2
ARQ531	Merck(ArQule)	P2
LOXO-305	Loxo / Lilly	P1/2



AS-0871: Development undergoing targeting <u>autoimmune diseases</u>

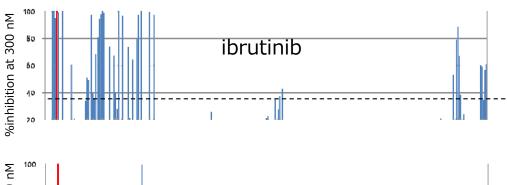
- Small molecule BTK inhibitor
- Non-covalent/reversible
- High kinase selectivity

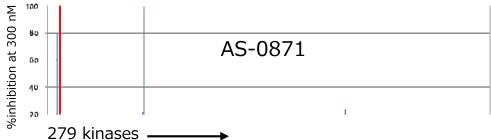
- Demonstrated significant efficacies in arthritis models
- Showed efficacy in systemic lupus erythematosus model
- CTA approved in February 2020
- ✓ GMP bulk production has been completed.
- ✓ GLP tox studies and safety studies have been completed.
- ✓ Clinical Trial Application (CTA) in Netherlands was approved in February 2020.
- ✓ Plans to initiate Phase 1 study in healthy volunteers in the 1H of 2020.

AS-0871: High Kinase Selectivity and Efficacy



◆ High kinase selectivity





◆ AS-0871 inhibits an allergic reaction

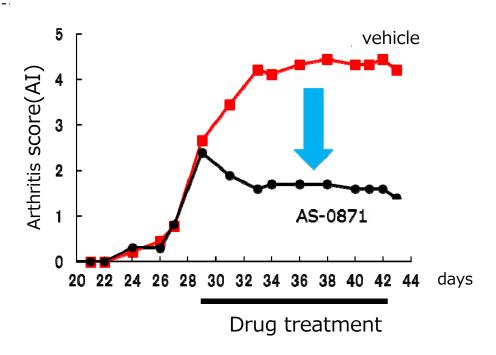




Vehicle

AS-0871

Therapeutic efficacy in Collageninduced arthritis (CIA) mice





AS-1763: Development undergoing targeting <u>blood cancer</u>

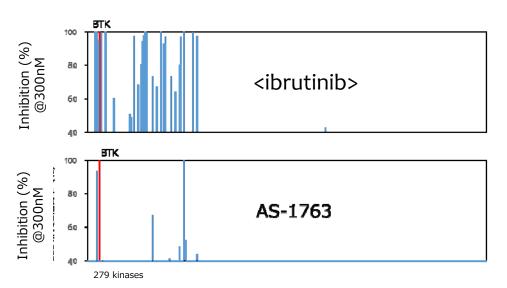
- Non-covalent/reversible
- High kinase selectivity
- Inhibits both BTK wild type and ibrutinib resistant BTK C481S mutants
- Displayed strong anti-tumor effects in lymphoma model
- Preclinical development undergoing with IND submission targeted in 2020
- Displayed efficacy in immuno-oncology model
- Potential applications for autoimmune diseases

✓ Preclinical development undergoing with IND submission targeted in 2020.

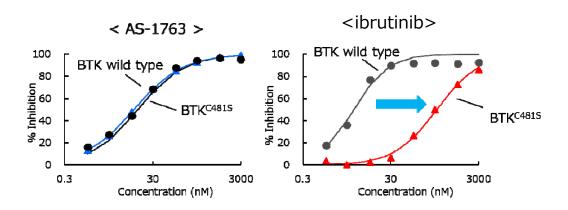
AS-1763: High Kinase Selectivity and Efficacy



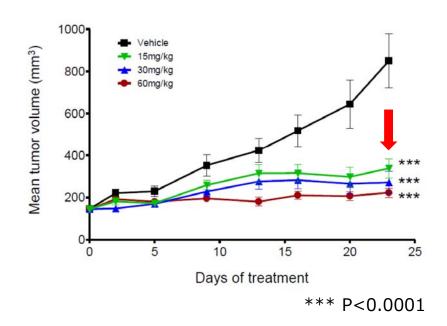
High kinase selectivity



◆ AS-1763 inhibits both WT and C481S mutant BTK enzymes



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





Drug Discovery Support

Drug Discovery Support FY2019 Key Highlights



- Drug Discovery Support business achieved sales of JPY1,079 million, the record high sales.
 - ✓ North America: +154.0% yoy
 - ✓ Other region including China: +38.3% yoy
- Successfully launched a new service.
 - ✓ A new cell-based assay service using NanoBRETTM technology developed by Promega was well received by the customers in North America and Europe. FY2019 sales beat our initial plan.
- Expanded lineup of kinase proteins and profiling service
 - √ 18 products were newly added to our unique biotinylated kinase lineup.
 - ✓ 2 Kinase targets including lipid kinases were newly added to our profiling service.



FY2019 Results

Consolidated Financial Results



(JPY million)	FY2018 Actual	FY2019 Actual	YoY Change	FY2019 Plan as of Jun. 25, 2019	FY2019 Plan as of Jan. 9, 2020	
Sales	754	3,207	+2,452 +3 2 5.0%	3,038	3,206	Upfront payment of JPY2,128M from Gilead.
Operating Profit/Loss	(1,144)	977	+2,122	313	968	Gross profits increased thanks to upfront payment.
Ordinary Profit/Loss	(1,159)	957	+2,116	279	951	
Net Profit/Loss	(1,210)	828	+2,038	214	791	
R&D Cost	1,140	1,281	+ 141 + 12.4%	1,808	1,296	Investment in preclinical studies.

Note 1: Rounded down to the nearest million yen.

Note 2: YoY change % for Operating Profit, Ordinary Profit, and Net Profit are not presented since losses were recorded in the previous period.

FY2019

Results by Business Segment



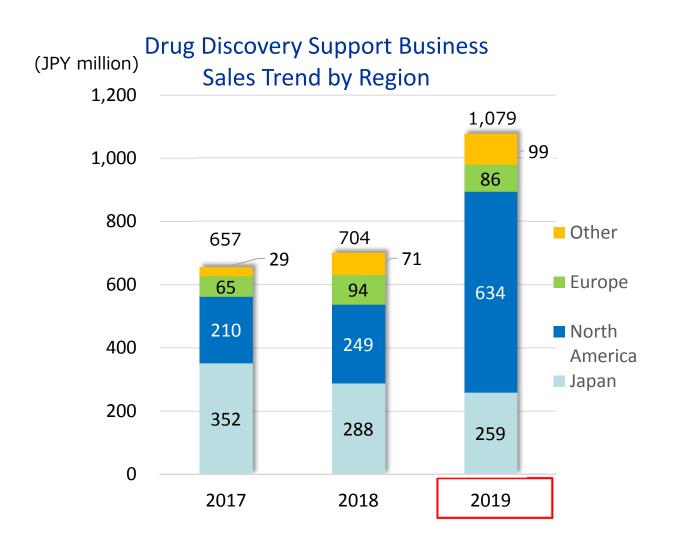
(JPY million)	FY2018 Actual	FY2019 Actual	YoY Change	FY2019 Plan as of Jun. 25, 2019	FY2019 Plan as of Jan. 9, 2020	
Total Sales	754	3,207	+2,452 +325%	3,038	3,206	
Drug Discovery Support	704	1,079	+374 +53. 2 %	938	1,078	Sales in the U.S. and China were strong.
Drug Discovery & Development	50	2,128	+2,078 +4156%	2,100	2,128	Upfront payment of JPY2,128M from Gilead.
Total Operating Profit/Loss	(1,144)	977	+2,122	313	968	
Drug Discovery Support	117	400	+283 +241%	247	403	Gross profit increased thanks to upbeat sales of internally developed products/services.
Drug Discovery & Development	(1,261)	577	+1,839	65	564	Investment in preclinical studies.

Note 1: Rounded down to the nearest million yen.

Note 2: YoY change % for Operating Profit is not presented since loss was recorded in the previous period.

FY2019 Sales Trend by Region Drug Discovery Support Business





- Japan: Decreased 10.2% YoY H1 sales decreased 30.9% YoY while H2 sales increased 13.7% YoY.
- North America: Increased 154.0% YoY Demand from new biotech companies for assay kits, kinase proteins, and profiling service were strong. Sales to Gilead also contributed.
- Europe: Decreased 8.3% YoY Sales of assay kits and profiling service were robust while kinase proteins were weak.
- Other: Increased 38.3%YoY Sales in China were strong. Expecting further growth from China.

Consolidated Balance Sheet



(JPY million)

				(JPY million)
	As of Dec. 31, 2018	As of Dec. 31, 2019	Change	Reason for changes
Current assets	1,671	5,274	+3,603	
Cash and deposits	1,355	4,915	+3,559	
Non-current Assets	98	101	+3	
Total assets	1,770	5,376	+3,606	
Current liabilities	400	1,055	+ 655	Unearned revenue +310, Accounts payable +141, Income taxes payable +101, current portion of longterm loans payable +66
Non-current liabilities	482	467	-14	Long term loans payable +4 Bonds payable -28
Total liabilities	882	1,523	+640	
Total net assets	887	3,853	+2,966	Capital stock and capital surplus +2,131, Retained earnings +828
Total liabilities and net assets	1,770	5,376	+3,606	
Shareholders' equity ratio	49.7%	71.5%		

 Shareholders' equity ratio
 49.7%
 71.5%

 BPS
 86.8 yen
 329.8 yen

 PBR
 10.4 x
 6.4 x

 Share price of Carna
 900 yen
 2,123 yen

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

Financing



Realize Drug Discovery Vision 2030 and become a Leading Drug Discovery company that continuously deliver innovative drugs

- Funds raised from warrants will be used to accelerate research and development.
 - ✓ Advance developments of two BTK inhibitors, AS-0871 and AS-1763.
 - ✓ Prime next wave of development programs.
 - ✓ Expand research pipeline.

Series 18th Subscription Rights to Shares

Series	Status of Exercise (as of the end of Jan.)	
Series 18th Subscription Rights to Shares	Total Number of shares exe Total value exercised	rcised 1,177,000 shares (72.4% of total rights) JPY2,142 million



Business Plan for FY2020

Anticipated Progress and Milestones through 2020



<Drug Discovery>

- AS-0871: Initiate Phase I study in H1.
- AS-1763: Submit an Initial New Drug (IND) application in H2.
- Bring one or more programs to preclinical stage.

<Drug Discovery Support>

- Achieve sales target of JPY1,036 million.
- Launch new products.
- Expand cell-based assay service using NanoBRETTM technology.

Drug Discovery: Vision 2030



Started drug discovery	Demonstrated strong capabilities in drug discovery	Maximize the value of pipelines	Continue delivering profits
2010~2015	2016~2020	2021~2025	2026~2030
 Established in- house research capability Established pipeline 	 Out-licensed multiple programs Initiate clinical trials 	 POC in human Strengthen cash position by milestone revenue 	 Drive sustainable revenue growth by milestones and royalties from multiple products



Leading Drug Discovery company that continuously deliver innovative drugs

<2020 R&D Plan>

- > AS-0871
 - ✓ FIH (1H2020)
- > AS-1763
 - ✓ IND submission (2020)
- Bring one or more programs to preclinical stage (2020)

<Revenue>

➤ Milestone payments and royalties from SRA141(AS-141), Sumitomo Dainippon Pharma, and Gilead

Maximum potential income from milestone payments: JPY86 bn in total + Sales royalties

Potential Revenue from new license deals including two BTK inhibitors

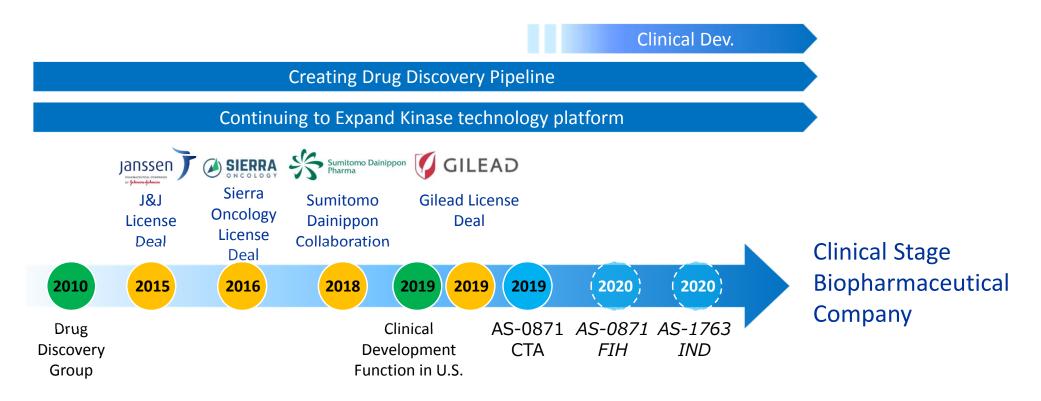
IND: Investigational New Drug

FIH: First in Human POC: Proof of Concept

Building a Sustainable Company



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



- ◆ Continuing to Expand Preclinical/Clinical Pipeline
- ◆ Established global clinical development capabilities to initiate clinical studies of two BTK inhibitor programs

FY2020 Financial Plan



(JPY million)	FY2019 Actual	FY2020 Plan	YoY Change	
Total Sales	3,207	1,036	-2,171 -67.7%	
Drug Discovery Support	1,079	1,036	-43 -4.0%	Not expecting bulk orders included in FY2019
Drug Discovery & Development	2,128	1	-2,128	Received upfront payment from Gilead in FY2019
Total Operating Loss	977	(1,779)	-2,757	
Drug Discovery Support	400	375	-24 -6.2%	
Drug Discovery & Development	577	(2,155)	-2,732	Investment in R&D
Ordinary Loss	957	(1,794)	-2,752	
Net Loss	828	(1,822)	-2,650	
R&D Cost	1,281	2,040	+758 +59.1%	Investment in clinical studies of AS-0871 and preclinical studies of AS-1763
Capex	42	54	+12	Equipment for R&D and information system

Note 1: Rounded down to the nearest million yen.

Drug Discovery Support: Sales Plan



(JPY mn)	FY2018 Actual	FY2019 Actual	FY2020 Plan
Drug Discovery Support	704	1,079	1,036
Kinase Proteins	314	385	340
Assay Development	27	310	338
Profiling & Screening	227	252	230
Cell-based Assay	4	19	19
Cell-based Assay (agent business)	109	78	84
Others	21	32	24

(JPY mn) 1200		rug Discover Sales Plan by				
1100		99	1,036	•		
1000		86	120			
900			77			
800				\vdash		
700	704					
600	71				Other	
	94	634	591			
500	240				Europe	
400	249				■ North	
300					America	
200					Japan	
100	288	259	246			
0						
FY2018 FY2019 FY2020 Plan						

Exchange rate(US\$):	110.45	109.30	105.0
% of Overseas sales:	59.1%	76.0%	76.0%

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





"Carna" is a goddess of Roman mythology who takes care of human health, protecting the human heart and other organs as well as everyday life, and is said to be the root for the word "cardiac."

The word "biosciences" is derived from the words 'biology' and 'life sciences.'

Carna Biosciences has created contemporary Carna goddess with protein kinase.

Carna Biosciences, Inc.

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