

Financial Results FY2018 Q2 (January to June 2018)

Carna Biosciences, Inc.



Stock Code : 4572

Company Overview



Company Name:	Carna Biosciences, Inc.	(As of June 30, 2018)
Representative:	Kohichiro Yoshino, President and CEO	
Established:	April 10, 2003 (a spin-off from Nippon Organon)	Bi
 IPO: Listed Market: Capital: 	March 25, 2008 (JASDAQ NEO) Tokyo Stock Exchange JASDAQ Growth 3,275 million yen	
 Number of shares issued: Number of 	9,622,600	
stockholders:Management and employees:	9,560 70	Business Support Center for Biomedical Research Activities in Kobe (BMA)
Address:	BMA 3F 1-5-5 Minatojima-Minamimachi, (Kobe Biomedical Innovation Cluster on Kobe Deloitte Touche Tohmatsu LLC	-
Auditor :	Defoitte Touche Tonmatsu LLC	
Business :	Drug Discovery and Development busine Discovery Support business	ss targeting kinases and Drug

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
- Secure funds to invest in our drug discovery programs



Our Business Model



Drug Discovery Support Service

Stable earnings from service business

 \Rightarrow Our strength



Production and sales of kinase proteins



Assay development Assay kits



Profiling and screening services

- Stable financial base
- Provide research tools



New technology
Recent drug discovery research trends

Drug Discovery and

Development

Research and develop new drugs in-house

⇒ Strategic investment to achieve accelerated growth

Internal R&D Drug Discovery & Development using proprietary kinase research platform focused on oncology and autoimmune diseases



Our commitment to quality



Drug Discovery Support Service

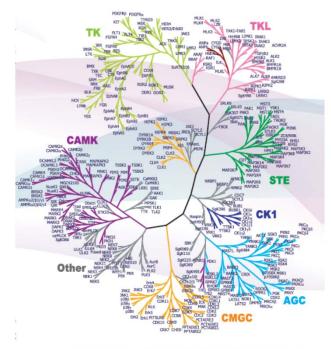
- Focus on quality (secure repeat customers)
- ✓ Unique products

Play an important role in kinase inhibitor drug discovery conducted by global pharmaceutical companies





- High quality kinase proteins
 - ✓ Lineup of approximately 450 products that are crucial to drug discovery research
- □ Accurate profiling service
 - Profiling service against approximately 340 kinases that well cover kinases in a living organism
- Assay kits and assay development that satisfy customer needs
- Cell-based assay services that provide further support to customers
 - ✓ ACD's Tyrosine Kinase Cell-Based Assay
 - ✓ CAI's ClariCELL™ Kinase Cell-Based Assays
 - ✓ NTRC's Oncolines[™], panel of cancer cell lines





Drug Discovery

- ✓ Develop innovative drug molecules from own research
- ✓ Proven track record in drug development including out-licensing of multiple programs
- ✓ Elite team with extensive experiences in large pharmas





YARNA BIOSCIE

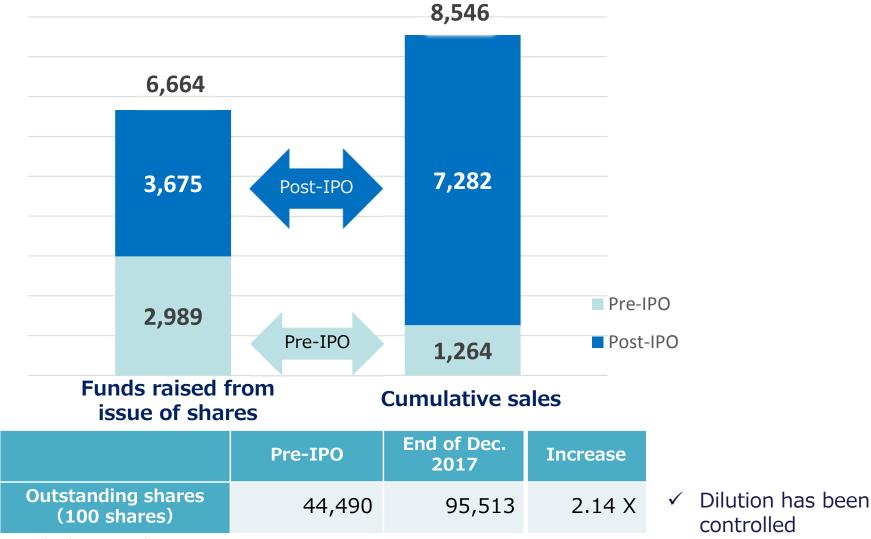


Advantage of Our Business Model: Reduced Investment Risk



 \checkmark Funds raised from business > Funds raised from issue of shares

Cumulative total since the formation of the company till the end of 2017 (JPY mn)



FY2018 Q2 Key Highlights



- National Cancer Center and Carna entered into a new research collaboration agreement for a novel drug target (May)
- Evotec AG and Carna announced to collaborate on Evotec's INDiGO platform (May)
- A restricted stock compensation plan was introduced to strengthen incentives for directors and employees to raise corporate value (May)
- Carna absorbed ProbeX, a former consolidated subsidiary, to streamline its operations (May)
- Establish the organization specializing in clinical development (July)
- <Intellectual Property>
- United States Patent and Trademark Office granted a patent for CDC7 inhibitor (April)
- Intellectual Property Office of Singapore granted a patent for BTK inhibitor (May)
- European Patent Office granted a patent for BTK inhibitor (June)

Building a Sustainable Company



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

Clinical Dev. Creating Drug Discovery Pipeline Continuing to Expand Kinase technology platform Sierra BTKi Clinical Spin-out J&J Oncology Portfolio Drug from N.V. Development AS-871 Discovery License License CB-1763 Group Organon IPO Group Deal Deal **Clinical Stage** Biopharmaceutical Company 2003 2008 2010 2015 2016 2017 2018 Continuing to Expand Preclinical/Clinical Pipeline Established clinical development capabilities to initiate in-house clinical studies



(JPY mn)	FY2017 Q2 Actual	FY2018 Q2 Actual	YoY Change	FY2018 Plan		
Sales	326	399	+72 (+22.1%)	1,190	 Received an upfront payment for a new joint research agreement. Sales of kinase proteins were robust. 	
Operating Loss	(291)	(436)	-144	(679)	Investment in R&D.	
Ordinary Loss	(297)	(443)	-145 (694)			
Net Loss	(316)	(489)	-173	(758)		
R&D Cost	270	468	+ 197 (+73.3%)	1,014	Investment in preclinical studies.	

Note 1: Rounded down to the nearest million yen.

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

Note 3: FY2018 plan was disclosed on February 9, 2018.

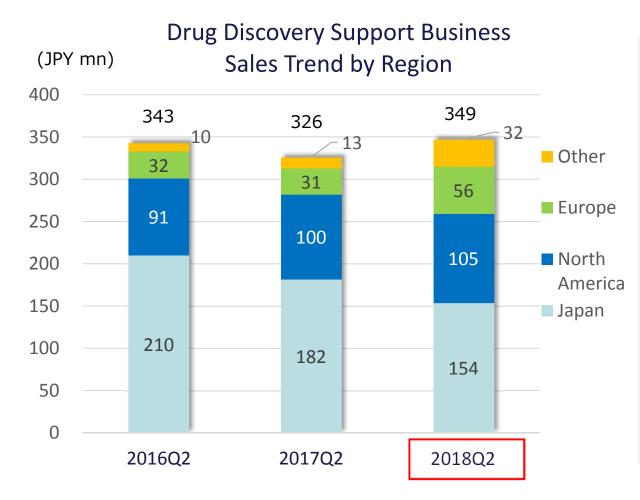


(JPY mn)	FY2017 Q2 Actual	FY2018 Q2 Actual	YoY Change	FY2018 Plan	Q2 Actual vs. FY Plan	
Total Sales	326	399	+72 (+22.1%)	1,190	33.5%	
Drug Discovery Support	326	349	+22 (+6.8%)	750	46.5%	Sales of kinase proteins were robust in Japan, EU, and in China.
Drug Discovery & Development		50	+50	440	11.4%	Received an upfront payment for a new joint research agreement.
Total Operating Loss	(291)	(436)	-144	(679)	—	
Drug Discovery Support	70	78	+8.4 (+12.1%)	150	52.4%	Profitability improved as sales of profitable kinase proteins were robust.
Drug Discovery & Development	(362)	(515)	-153	(829)		Investment in preclinical studies.

Note 1: Rounded down to the nearest million yen.

Note 2: YoY change % and comparison to FY2018 plan for Operating Loss are not presented since losses were recorded. Note 3: FY2018 plan was disclosed on February 9, 2018.





- Japan: Decreased 15.3% YoY Sales to Ono Pharmaceutical were weak while sales to other customers remained the same as the previous year.
- North America: Increased 5.5% YoY Profiling and screening services were robust while kinase proteins were weak.
- Europe: Increased 81.5% YoY
 <u>Sales of kinase proteins were robust.</u>
- Other: Increased 145.8%YoY
 <u>Sales of kinase proteins increased</u> significantly in China.

(Note) Sales to Ono Pharmaceuticals FY2017Q2 ··· 86 mn yen FY2018Q2 ··· 58 mn yen

Consolidated Balance Sheet



(JPY mn)

	As of Dec. 31, 2017	As of Jun. 30, 2018	Change	Reason for changes		
Current assets	2,134	1,842	-291			
Cash and deposits	1,856	1,519	-336			
Other	278	322	+44	Raw materials and supplies +29, Accounts receivable-trade -18		
Non-current Assets	56	116	+60			
Total assets	2,190	1,959	-231			
Current liabilities	341	390	+48	Current portion of long-term loans payable +94, Account payable-other -38		
Non-current liabilities	470	583	+113	Long term loans payable +127、 Bonds payable -14		
Total liabilities	812	974	+162			
Total net assets	1,377	984	-393	Retained earnings -489		
Total liabilities and net assets	2,190	1,959	-231			
Shareholders' equity ratio	62.2%	49.5%				
BPS	142.68 yen	100.73 yen				
PBR	7.91 x	13.09 x				
Share price of Carna Biosciences	1,128 yen	1,319 yen	n Note: Share price of Carna Biosciences is based on the close price of JASDAQ growth			

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Pipeline Status

CARNA BIOSCIENCES

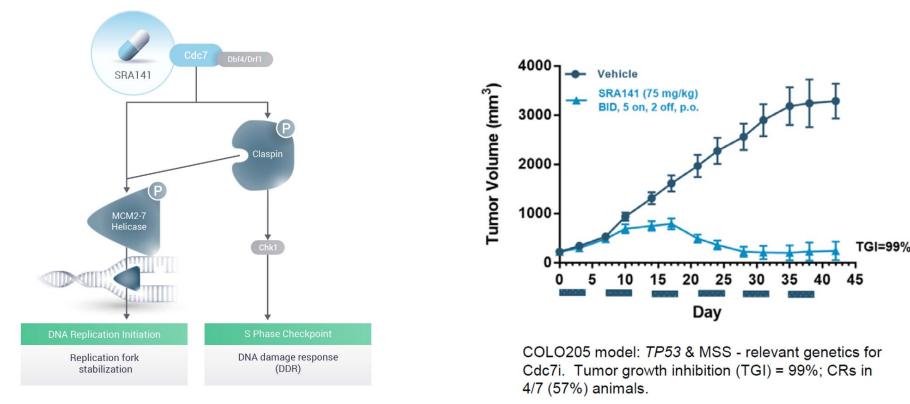
Compound	Target	Target Indication	Development Phase					
			Discovery	Preclinical	PhI	PhII	PhIII	Partner/ Collaboration partner
SRA141 (AS-141)	CDC7/ASK	Cancer						SIERRA ONCOLOGY
AS-871	ВТК	Autoimmune Diseases						
CB-1763	ВТК	Blood Cancer Immuno-Oncology						
Small Molecule	Wnt-signal	Cancer Immuno-Oncology						🥙 National Cancer Center Japan
Small Molecule	Kinase	Psychiatry & neurology						Sumitomo Dainippon Pharma
Small Molecule	TGFβ signaling	Blood Cancer Immuno-Oncology						HIROSHIMA UNIVERSITY
Small Molecule	Kinase	Autoimmune Diseases						
Small Molecule	N/A	Malaria						KITASATO UNIVERSITY
Small Molecule	DGK	Immuno-Oncology						
Small Molecule	Undisclosed	Cancer						🧐 National Cancer Center Japan

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

 $\checkmark\,$ Sierra is advancing the research to initiate clinical study, anticipating to submit an IND in H2 2018.

ARNA BIOSCIEN

✓ Recent studies demonstrated that SRA141 significantly inhibits tumor growth in AML MV4-11 rat xenograft model and colorectal COLO-205 rat xenograft model. In AML model, some rats were cured completely and in colorectal model, complete tumor regressions were observed for over half of rats. Phase 1/2 clinical program to be focused on colorectal cancer.



Source: Sierra Oncology

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AS-871 : Non-covalent BTK Inhibitor targeting autoimmune diseases



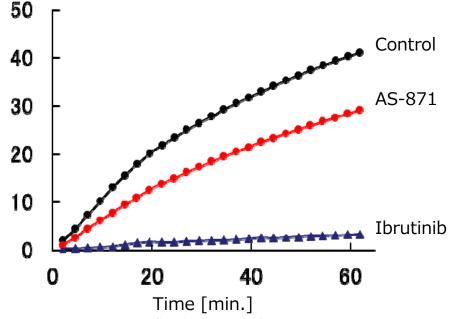
- ✓ GMP bulk material production is initiated for Phase I clinical study.
- ✓ Preparing preclinical safety studies under GLP conditions.

AS-871 : Development undergoing targeting <u>autoimmune diseases</u>

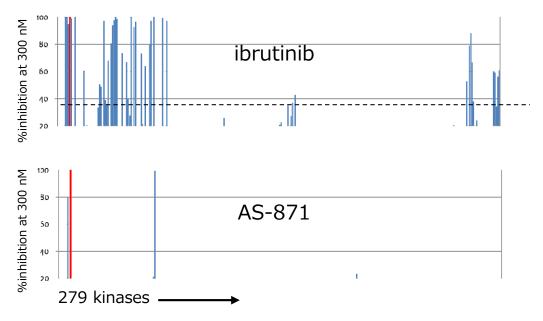
- Small molecule BTK inhibitor
- Non-covalent/reversible
- High kinase selectivity

- Demonstrated significant efficacies in arthritis models
- Showed efficacy in systemic lupus erythematosus model
- Preclinical development undergoing with IND submission targeted in the first half of 2019

Non-covalent/Reversible inhibition



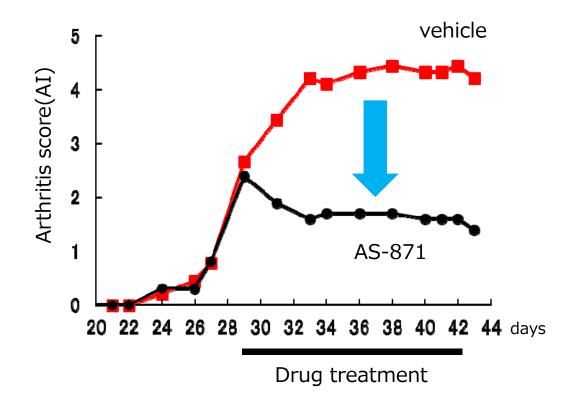
High kinase selectivity



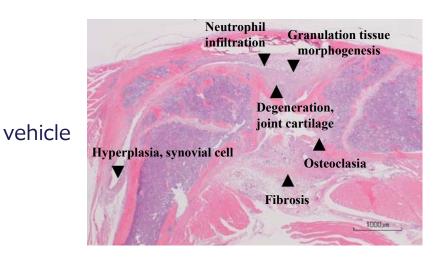
AS-871 : Non-covalent BTK Inhibitor targeting autoimmune diseases

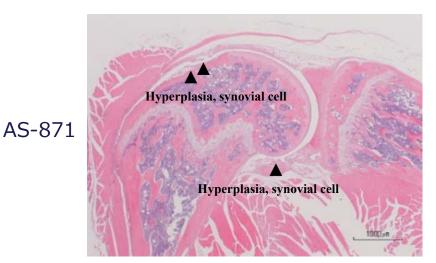






Histopathology of knee joints



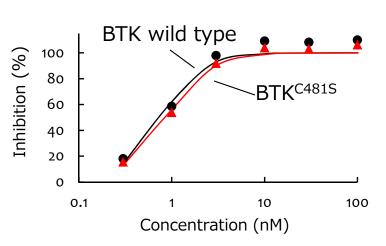


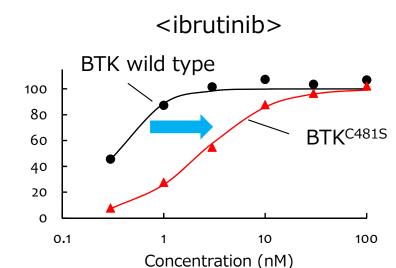
CB-1763:Next Generation BTK Inhibitor Targeting Blood Cancer

✓ We are accelerating the preclinical development of CB-1763 by leveraging Evotec AG's INDiGO platform.

CB-1763 : Development undergoing targeting blood cancer Non-covalent/reversible High kinase selectivity Inhibits both BTK wild type and ibrutinib resistant BTK C481S mutants Preclinical development undergoing with IND submission targeted in the first half of 2019 Potential applications for autoimmune diseases

CB-1763 inhibits both WT and C481S mutant BTK enzymes





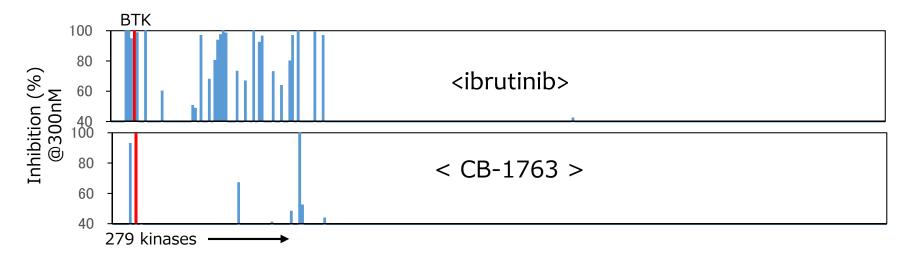
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< CB-1763 >

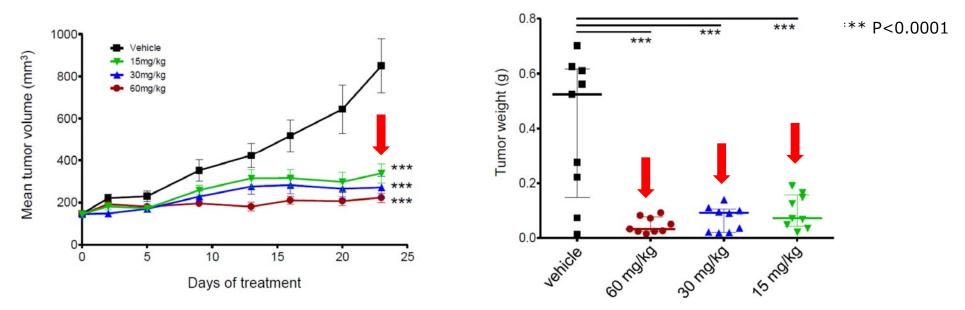
CB-1763:Next Generation BTK Inhibitor Targeting Blood Cancer



• CB-1763 is a highly selective inhibitor

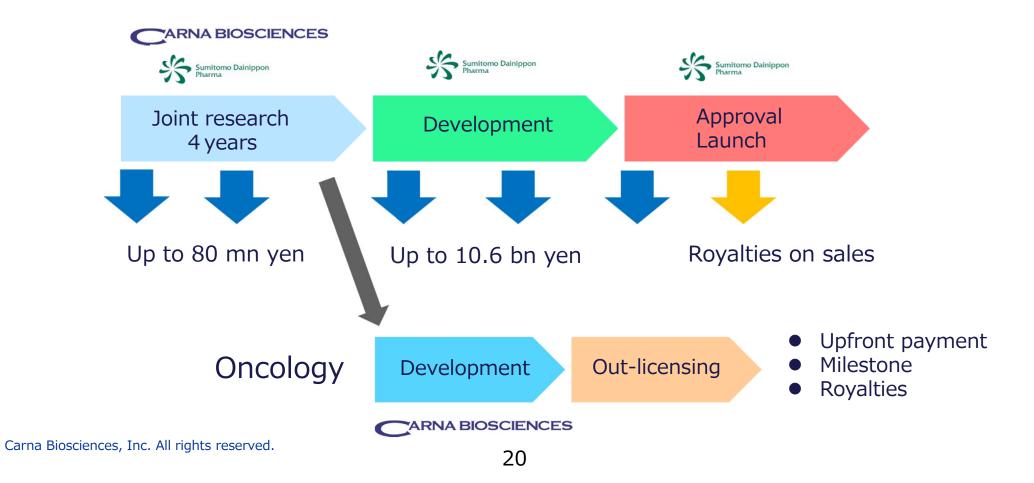


• CB-1763 significantly inhibits tumor growth in a B-cell lymphoma mouse model



Joint Research agreement with Sumitomo Dainippon Pharma

- ✓ Carana and Sumitomo Dainippon Pharma are conducting joint research based on the agreement signed in March 2018 on joint research with the aim of discovering novel kinase inhibitors for psychiatric and neurological disorders as well as on the subsequent development and commercialization of such therapeutic agent(s).
- ✓ Carna has received an upfront payment in accordance with the agreement. Carna will receive up to approximately 10.6 billion yen in total as an upfront payment and milestone payments on development and sales.



Summary of Drug Discovery Support Business



- ✓ Overall sales and operating profit of the Drug Discovery Support Business increased thanks to the robust sales in Europe and the significant sales increase in China.
- ✓ In North America, profiling services were robust. Carna will continue seeking new customers, aiming to expand market share further.
- ✓ In order to establish a strong presence in China where drug discovery research is rapidly expanding, Carna will continue strengthening relationship with our distributor and promoting our products and services aggressively.
- In Europe, our agent promoted our products and services actively to the existing customers, which contributed to the strong sales. We will continue working closely with the agent to increase sales activities further.
- \checkmark We continued to focus our marketing efforts on expanding sales of DGK assay kits.
- \checkmark Preparing to launch a new cell-based assay service by the year end.





"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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