

# Financial Results

## FY2018 Q3

(January to September 2018)

# Carna Biosciences, Inc.



**Stock Code : 4572**

- **Established the clinical development team to initiate clinical trials of Carna's drug candidates (July)**
- Published the medicinal chemistry and its biological data to discover a series of novel BTK inhibitors in Journal of Medicinal Chemistry (September)
- European Patent Office granted a patent for Wnt-signal inhibitor (September)

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October~

- **Sierra Oncology successfully completed the investigational New Drug Application (IND) filing process with FDA (November)**

# FY2018 Q3 Consolidated Financial Results



(JPY mn)	FY2017 Q3 Actual	FY2018 Q3 Actual	YoY Change	FY2018 Plan	
Sales	483	<b>586</b>	+103 (+21.4%)	1,190	<ul style="list-style-type: none"> <li>- Sales were robust in the U.S., Europe, and China.</li> <li>- Received an upfront payment for a new joint research agreement.</li> </ul>
Operating Loss	(477)	<b>(762)</b>	-285	(679)	Investment in R&D.
Ordinary Loss	(486)	<b>(773)</b>	-286	(694)	
Net Loss	(508)	<b>(822)</b>	-314	(758)	
R&D Cost	445	<b>791</b>	+345 (+77.6%)	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.

# FY2018 Q3 Results by Business Segment



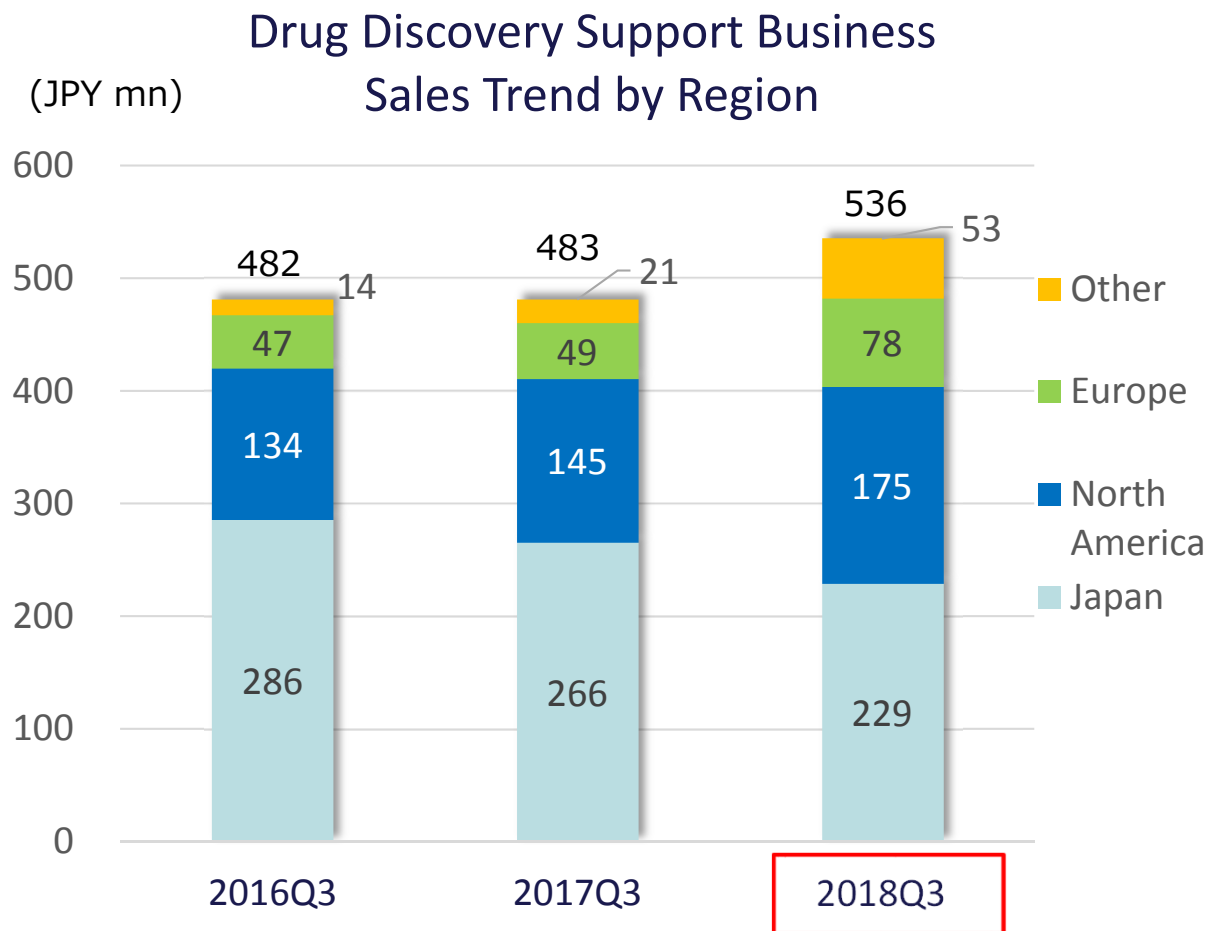
(JPY mn)	FY2017 Q3 Actual	<b>FY2018 Q3 Actual</b>	YoY Change	FY2018 Plan	Q3 Actual vs. FY Plan	
Total Sales	483	<b>586</b>	+103 (+21.4%)	1,190	49.3%	
Drug Discovery Support	483	<b>536</b>	+53 (+11.1%)	750	71.5%	Sales were robust in the U.S., Europe, and China. Kinase proteins and cell-based assay services were especially strong.
Drug Discovery & Development	—	50	+50	440	11.4%	Received an upfront payment for a new joint research agreement.
Total Operating Loss	(477)	<b>(762)</b>	-285	(679)	—	
Drug Discovery Support	99	<b>114</b>	+14 (+14.8%)	150	76.0%	Profitability improved as sales of profitable kinase proteins were robust.
Drug Discovery & Development	(576)	<b>(876)</b>	-300	(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.

# FY2018 Q3 Sales Trend by Region Drug Discovery Support Business



- Japan: Decreased 13.7% YoY  
Sales to Ono Pharmaceutical were weak while sales to other customers were steady, exceeding the previous year.
- North America: Increased 20.3% YoY  
Cell-based assay services were strong.
- Europe: Increased 57.9% YoY  
Sales of kinase proteins were robust.
- Other: Increased 147.7% YoY  
Sales of kinase proteins increased significantly in China.

(Note) Sales to Ono Pharmaceuticals  
FY2017Q3 ... 117 mn yen  
FY2018Q3 ... 76 mn yen

# Consolidated Balance Sheet



(JPY mn)

	As of Dec. 31, 2017	As of Sep. 30, 2018	Change	Reason for changes
Current assets	2,134	1,523	-611	
Cash and deposits	1,856	1,144	-712	
Other	278	379	+101	Accounts receivable-trade +26, Raw materials and supplies +25
Non-current Assets	56	106	+49	
Total assets	2,190	1,629	-561	
Current liabilities	341	408	+66	Current portion of long-term loans payable +69
Non-current liabilities	470	525	+54	Long term loans payable +82, Bonds payable -28
Total liabilities	812	933	+120	
Total net assets	1,377	695	-681	Retained earnings -822, Capital stock and capital surplus +141
Total liabilities and net assets	2,190	1,629	-561	
Shareholders' equity ratio	62.2%	41.8%		
BPS	142.68 yen	70.56 yen		
PBR	7.91 x	21.7 x		
Share price of Carna Biosciences	1,128 yen	1,534 yen		

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

# Pipeline Status



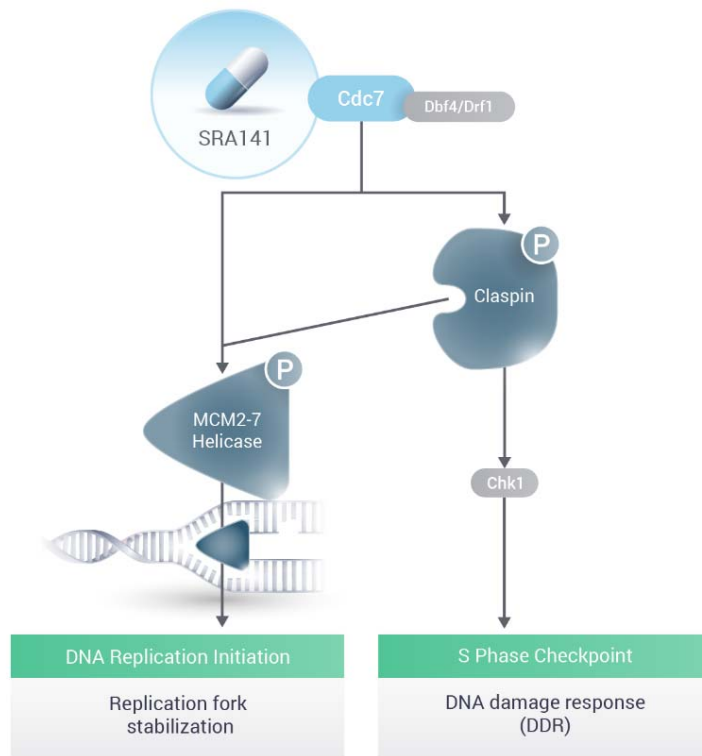
Compound	Target	Indication	Development Phase					Partner/ Collaboration partner
			Discovery	Preclinical	PhI	PhII	PhIII	
SRA141 (AS-141)	CDC7/ASK	Cancer		IND completed				SIERRA ONCOLOGY
AS-0871	BTK	Autoimmune Diseases						
AS-1763*	BTK	Blood Cancer Immuno-Oncology						
Small Molecule	Wnt-signal	Cancer 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

\*The compound name "CB-1763" has been changed into the official development code "AS-1763."

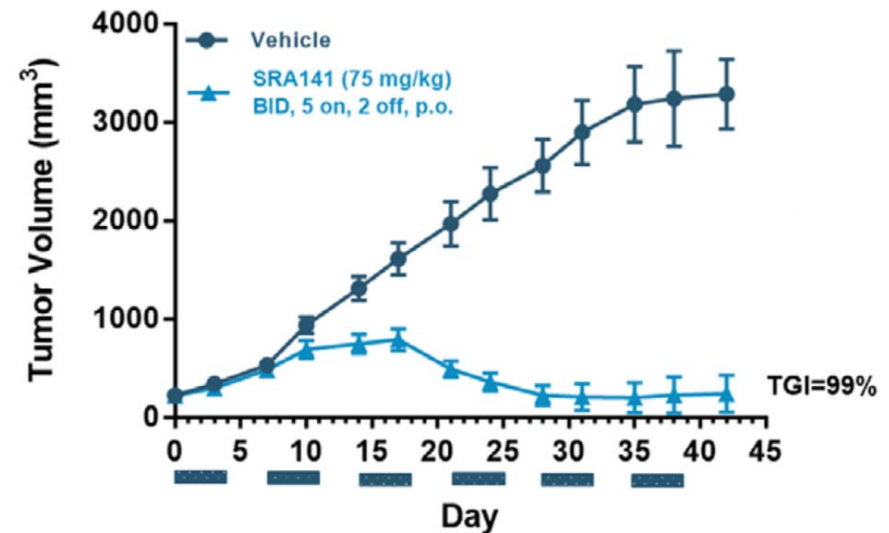
# SRA141 : CDC7 Inhibitor Targeting Cancer (Out-licensed to Sierra Oncology)



- ✓ 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 mn) upon initiation of Phase 1 study.
- ✓ Sierra will present the result of preclinical study for SRA141 at EORTC-NCI-AACR Symposium on Molecular Targets and Cancer Therapeutics on November 13-16.



Source: Sierra Oncology



COLO205 model: *TP53* & *MSS* - relevant genetics for *Cdc7i*. Tumor growth inhibition (TGI) = 99%; CRs in 4/7 (57%) animals.

Source: Sierra Oncology



# AS-0871 : Non-covalent BTK Inhibitor targeting autoimmune diseases

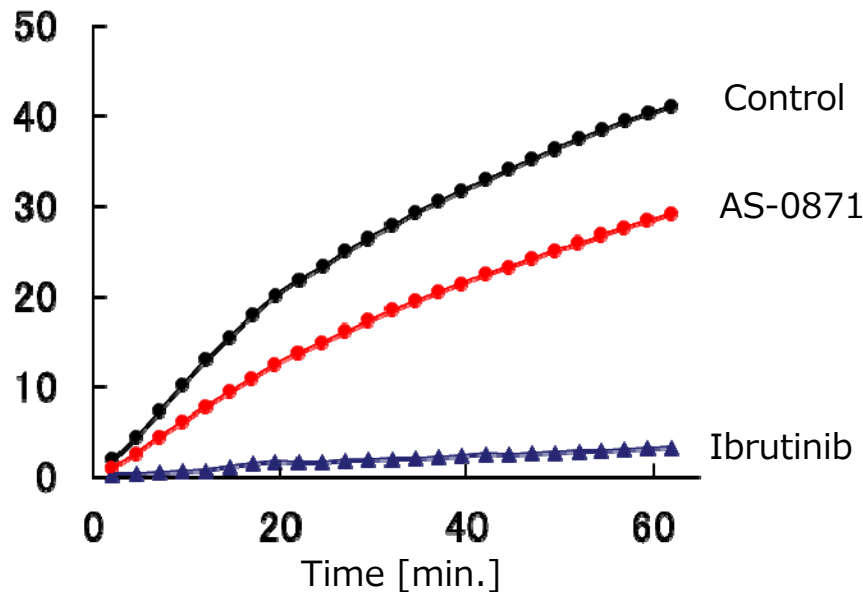


- ✓ Non-GMP bulk production for GLP tox studies has been completed.
- ✓ GMP bulk material production for Phase I clinical study is ongoing.
- ✓ No serious toxicity has been observed in single oral dose range-finding toxicity study in rodent even at the highest dose.

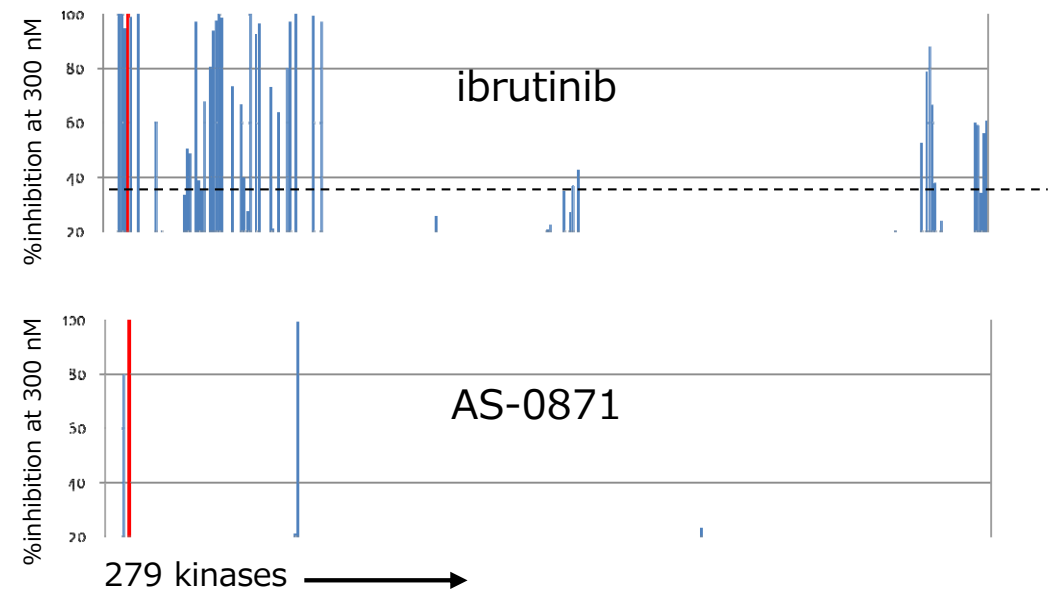
## AS-0871 : Development undergoing targeting autoimmune diseases

- 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 CTA submission targeted in the first half of 2019

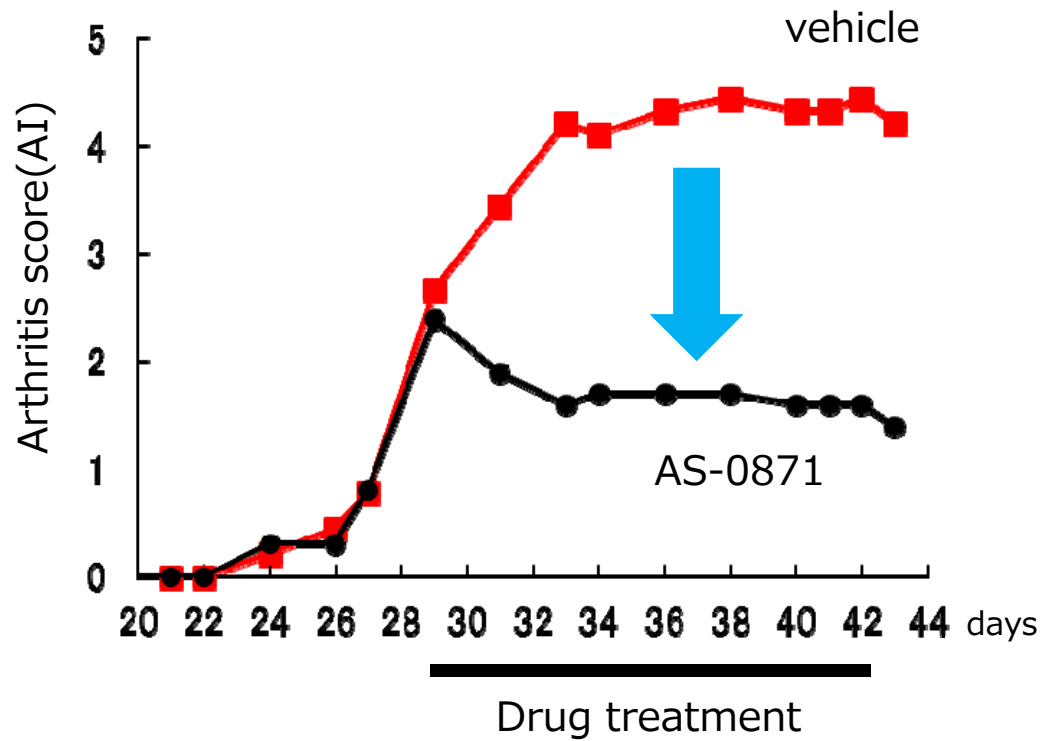
### ◆ Non-covalent/Reversible inhibition



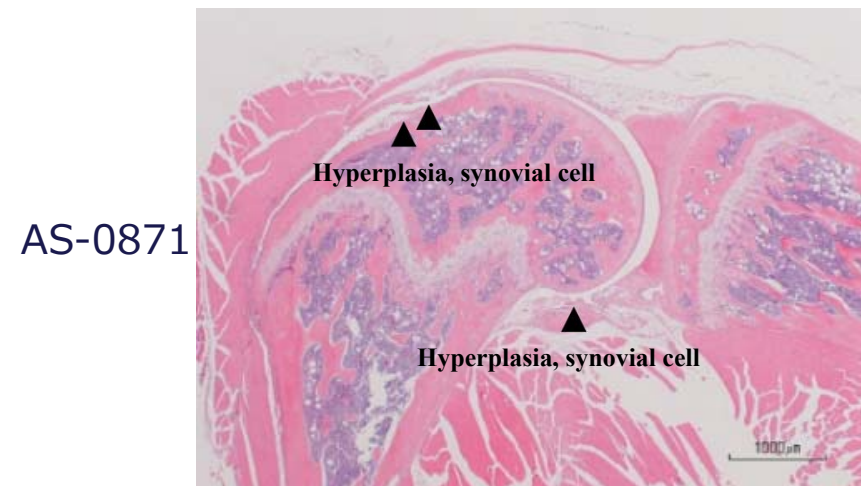
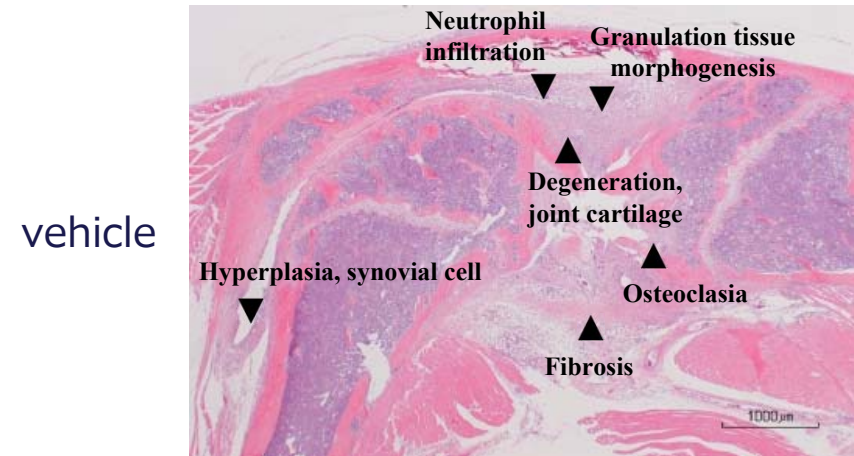
### ◆ High kinase selectivity



## Therapeutic efficacy in Collagen-induced arthritis (CIA) mice



## Histopathology of knee joints



# AS-1763:Next Generation BTK Inhibitor Targeting Blood Cancer

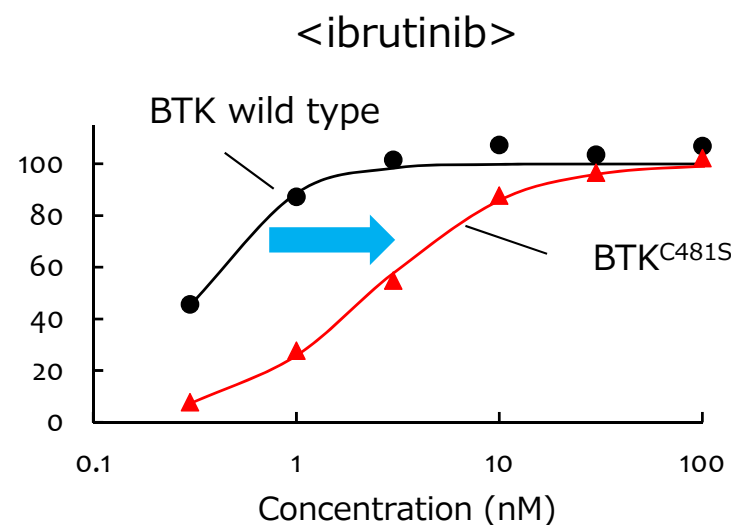
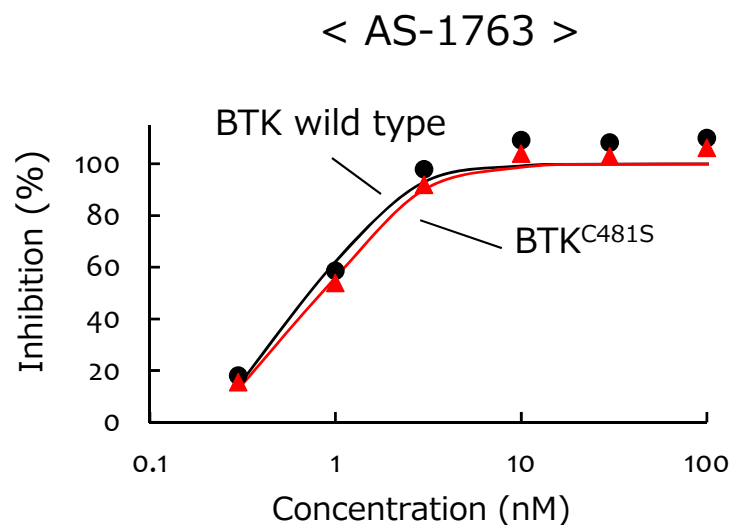


- ✓ We are accelerating the preclinical development of AS-1763 by leveraging Evotec AG's INDiGO platform.
- ✓ API process development was completed.
- ✓ The compound name "CB-1763" has been changed into the official clinical development code "AS-1763" as the IND-enabling studies have been progressed

## AS-1763 : Development undergoing targeting blood cancer

- 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 the second half of 2019
- Potential applications for autoimmune diseases

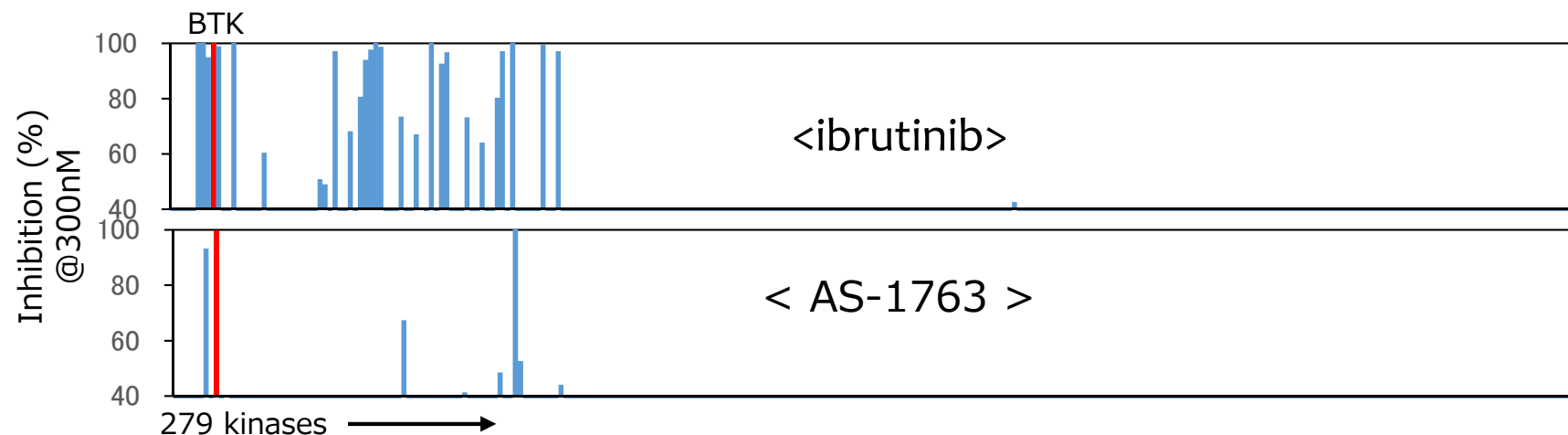
- AS-1763 inhibits both WT and C481S mutant BTK enzymes



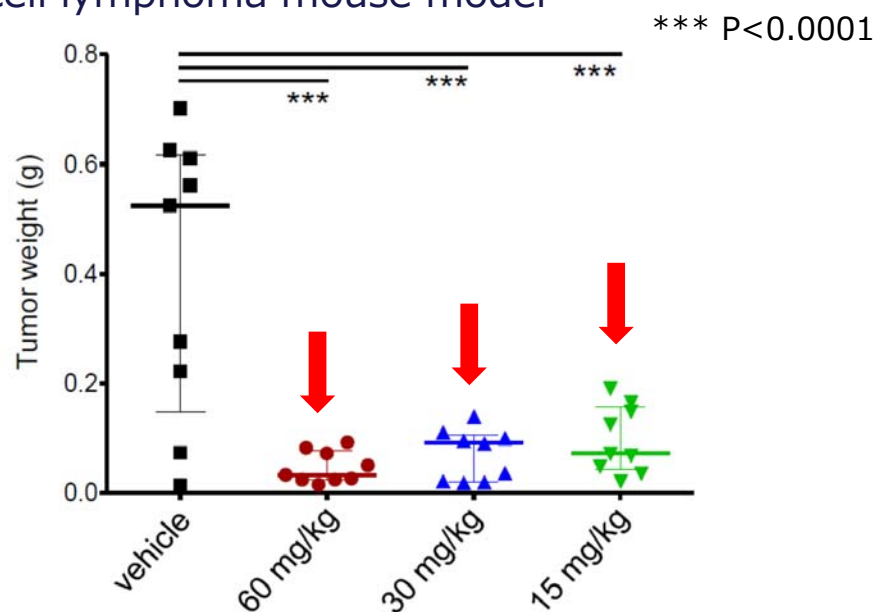
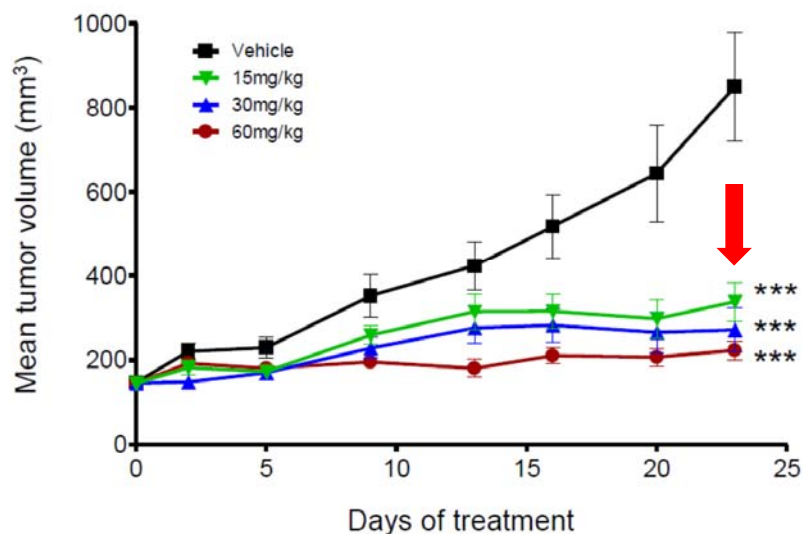
# AS-1763: Next Generation BTK Inhibitor Targeting Blood Cancer



- AS-1763 is a highly selective inhibitor



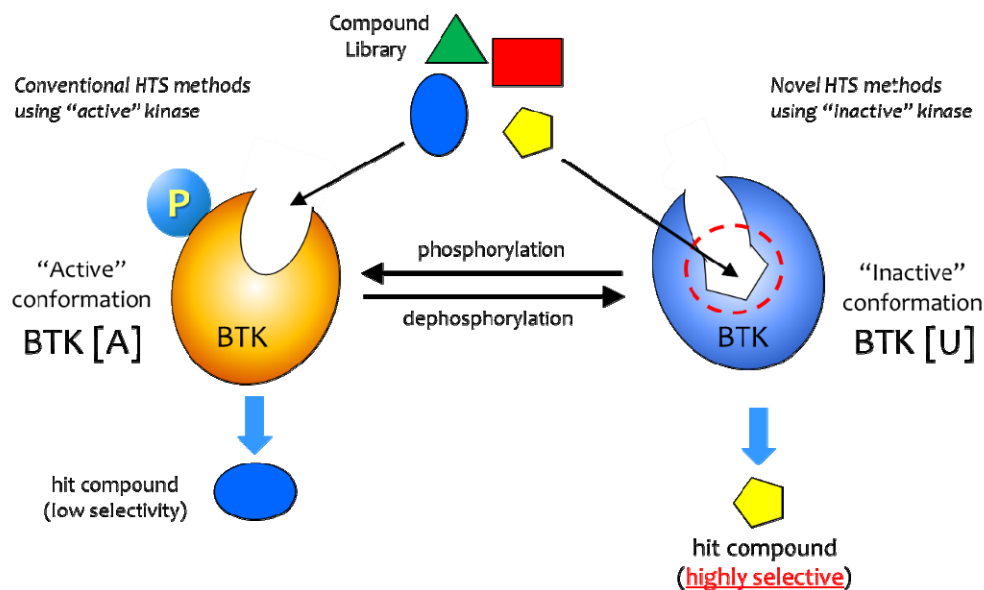
- AS-1763 significantly inhibits tumor growth in a B-cell lymphoma mouse model



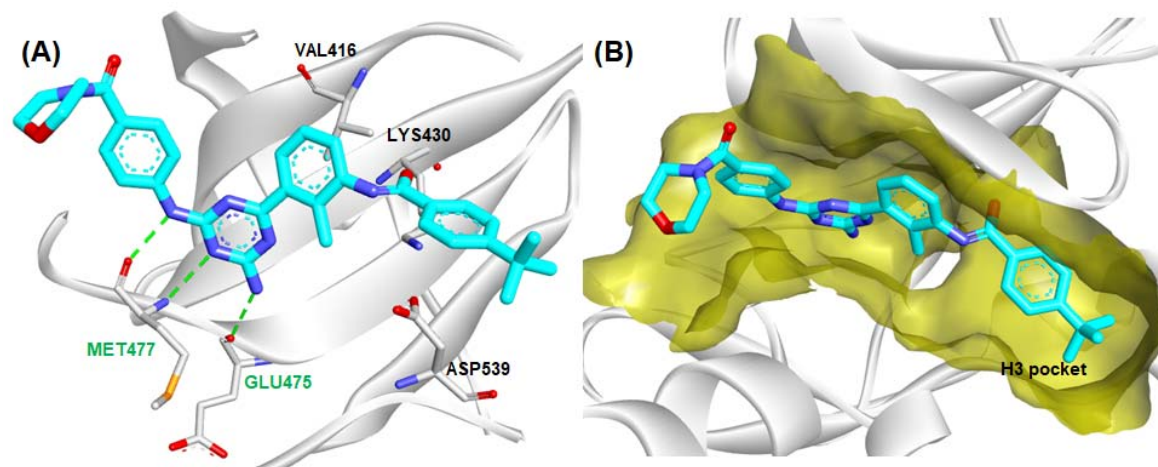
# Published a Research Paper on Discovery of BTK Inhibitor

- ✓ The medicinal chemistry and its biological data to discover a series of novel BTK inhibitors have been published in Journal of Medicinal Chemistry.
- ✓ In order to generate a highly selective non-covalent BTK inhibitor, the drug discovery team developed novel drug discovery technology using two conformationally different BTK proteins, an activated form of BTK (BTK[A]) and an unactivated form of BTK (BTK[U].)
- ✓ Using this novel technology, the drug discovery team successfully identified a new lead compound showing a stronger inhibitory potency for BTK[U] than for BTK[A].
- ✓ Subsequent lead optimization led to the discovery of AS-0871 having strong inhibitory potency for BTK with high kinase selectivity in a non-covalent manner.

Novel screening method using an unactivated conformation of kinase

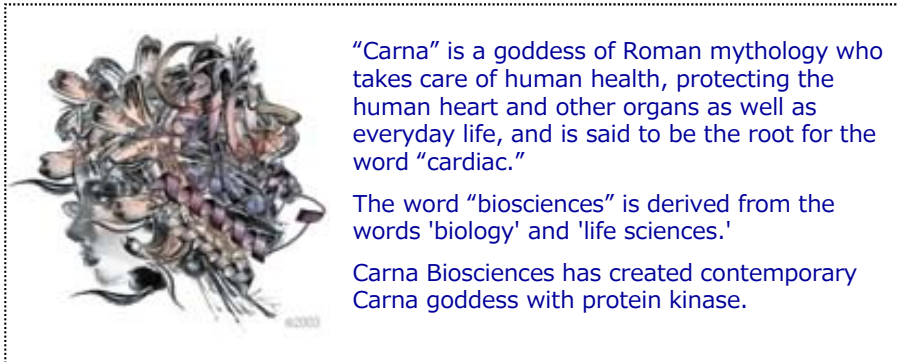


X-ray co-crystal structure of BTK complexed with a lead compound



- ✓ Overall sales and operating profit of the Drug Discovery Support Business increased thanks to the robust sales in North America, Europe, and China.
- ✓ In North America, sales from new biotech companies are expanding. 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.
- ✓ 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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