

# Financial Results

## FY2021 Q1

(January to March 2021)

## Carna Biosciences, Inc.



**Stock Code : 4572**

- Completed PMDA review for a clinical trial notification to initiate clinical trial of AS-0141 in Japan. (February)
- A Clinical Trial Application (CTA) was approved in the Netherlands to initiate clinical trial of AS-1763. (February)
- Initiated dosing in a FIH Phase 1 study of BTK inhibitor AS-1763. (April)

(JPY mn)	FY2020 Q1 Actual	FY2021 Q1 Actual	YoY Change	FY2021 Plan	
Sales	335	<b>231</b>	-103 -31.0%	923	-On track to achieve FY sales plan. -Received an upfront payment from licensing in Q1 FY2020.
Operating Profit/Loss	(165)	<b>(291)</b>	-126	(1,811)	
Ordinary Profit/Loss	(168)	<b>(284)</b>	-115	(1,816)	
Net Profit/Loss	(184)	<b>(286)</b>	-101	(1,825)	
R&D Cost	328	<b>357</b>	+28 +8.7%	1,981	Investment in clinical studies.

Note 1: Rounded down to the nearest million yen.

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

Note 3: FY2021 plan was disclosed on February 12, 2021.

(JPY mn)	FY2020 Q1 Actual	FY2021 Q1 Actual	YoY Change	FY2021 Plan	vs. FY Plan	
Total Sales	335	<b>231</b>	-103 -31.0%	923	25.1%	
Drug Discovery Support	282	<b>231</b>	-50 -18.0%	923	25.1%	On track to achieve FY sales plan.
Drug Discovery & Development	53	—	-53	—	—	Received an upfront payment from licensing in Q1 2020.
Total Operating Profit/Loss	(165)	<b>(291)</b>	-126	(1,811)	—	
Drug Discovery Support	132	<b>88</b>	-44 -33.3%	207	42.4%	On track vs. FY plan.
Drug Discovery & Development	(297)	<b>(379)</b>	-82	(2,019)	—	Investment in clinical studies.

Note 1: Rounded down to the nearest million yen.

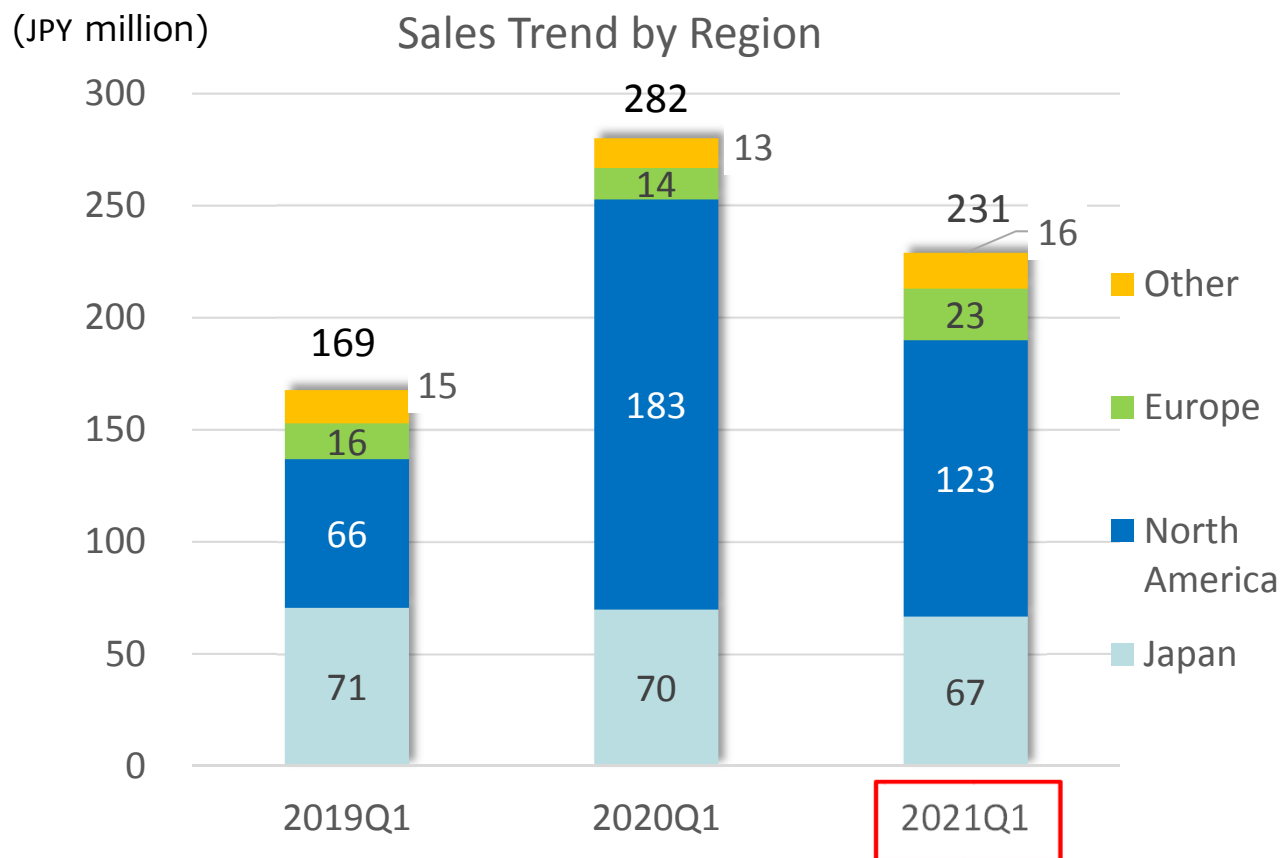
Note 2: YoY change % for consolidated operating profit/loss and drug discovery and development operating profit/loss are not presented since losses were recorded.

Note 3: FY2021 plan was disclosed on February 12, 2021.

# FY2021 Q1 Sales Trend by Region Drug Discovery Support Business



Drug Discovery Support Business  
Sales Trend by Region



- Japan: Decreased 4.7% YoY  
Sales of kinase proteins and profiling service were robust.
- North America: Decreased 32.3% YoY  
Sales decreased compared to an upbeat sales in Q1 FY2020, but on track vs. plan. Sales to Gilead contributed.
- Europe: Increased 61.6% YoY  
Profiling service and NanoBRET assay service were robust.
- Other: Increased 20.4% YoY  
Sales in China continued show an recovery.

# Consolidated Balance Sheet











(JPY mn)

	As of Dec. 31, 2020	As of Mar. 31, 2021	Change	Reason for changes
Current assets	4,708	4,108	-599	
Cash and deposits	4,299	3,734	-564	
Non-current Assets	127	119	-7	
Total assets	4,835	4,228	-607	
Current liabilities	727	442	-285	Accounts payable -194
Non-current liabilities	284	235	-48	Long term loans payable -35 Bonds payable -14
Total liabilities	1,011	677	-333	
Total net assets	3,824	3,550	-273	Retained earnings -286
Total liabilities and net assets	4,835	4,228	-607	

Shareholders' equity ratio	79.0%	83.9%
BPS	308.0 yen	286.0 yen
PBR	3.9 x	4.7 x
Share price of Carna	1,212 yen	1,354 yen







Note: Share price is the closing price of the term end.

## <Oncology>

Compound	Target	Indication	Discovery/ Preclinical	Clinical	Partner
AS-0141	CDC7/ASK	Cancer		Ph1 planned in H1 2021	
Small Molecule	Kinase	Immuno-Oncology			
AS-1763	BTK	Blood Cancer Immuno-Oncology			
Small Molecule	ALK5	Blood Cancer Immuno-Oncology			
Small Molecule	CDK1	Cancer			

\*Greater China only

## <Other Therapeutic Areas>

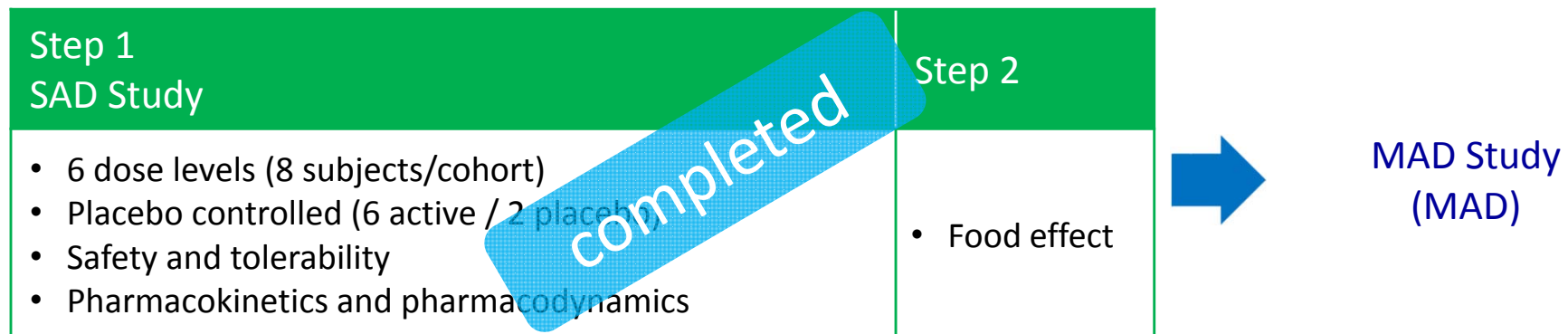
Compound	Target	Indication	Discovery/ Preclinical	Clinical	Partner
Small Molecule	Kinase	Psychiatry & neurology			
AS-0871	BTK	Immune-inflammatory diseases			
Small Molecule	N/A	Malaria			
Small Molecule	STING	Immune-inflammatory diseases			

➤ We are actively pursuing early discovery programs to create next wave of pipeline.

## AS-0871 : Development for Autoimmune Diseases

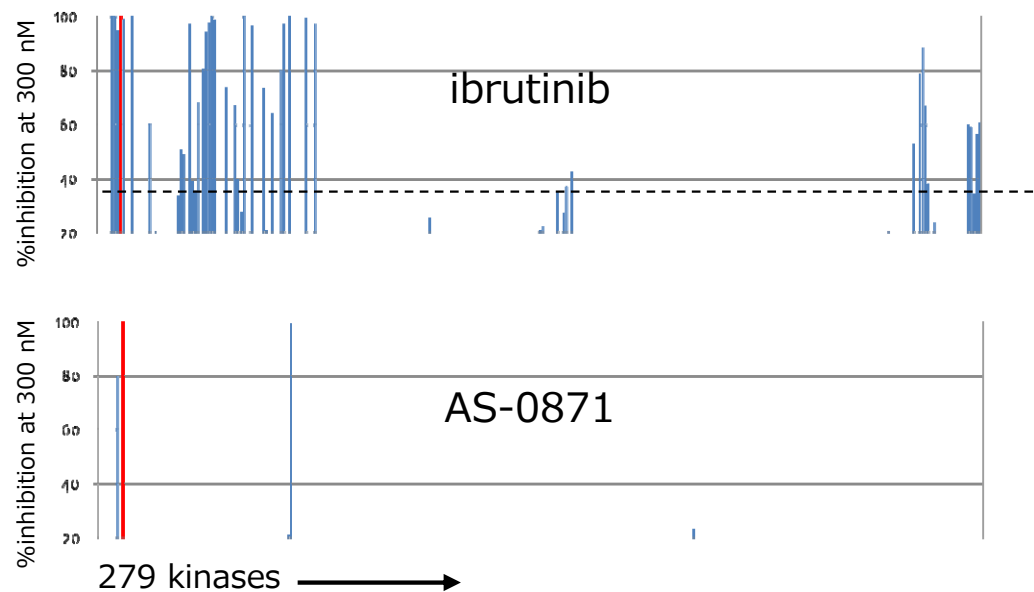
- Small molecule BTK inhibitor
- Non-covalent/reversible
- High kinase selectivity
- Orally available
- Demonstrated significant efficacies in arthritis models
- Showed efficacy in systemic lupus erythematosus model
- Initiated P1 study in August 2020 and completed dosing in SAD studies

- ✓ Phase 1 Single Ascending Dose (SAD) study in healthy volunteers was initiated in August 2020 in the Netherlands, finding AS-0871 was well-tolerated without any safety concerns at all dose levels.
- ✓ Blood samples to assess pharmacodynamic effects were analyzed for evaluation of the B-cell and basophil responses. Administration of AS-0871 at 100mg or above resulted in strong inhibition of B-cell and basophil activation.
- ✓ Multiple Ascending Dose (MAD) study using new drug formulation is planned in H2.





## ◆ High kinase selectivity



## ◆ AS-0871 inhibits an allergic reaction

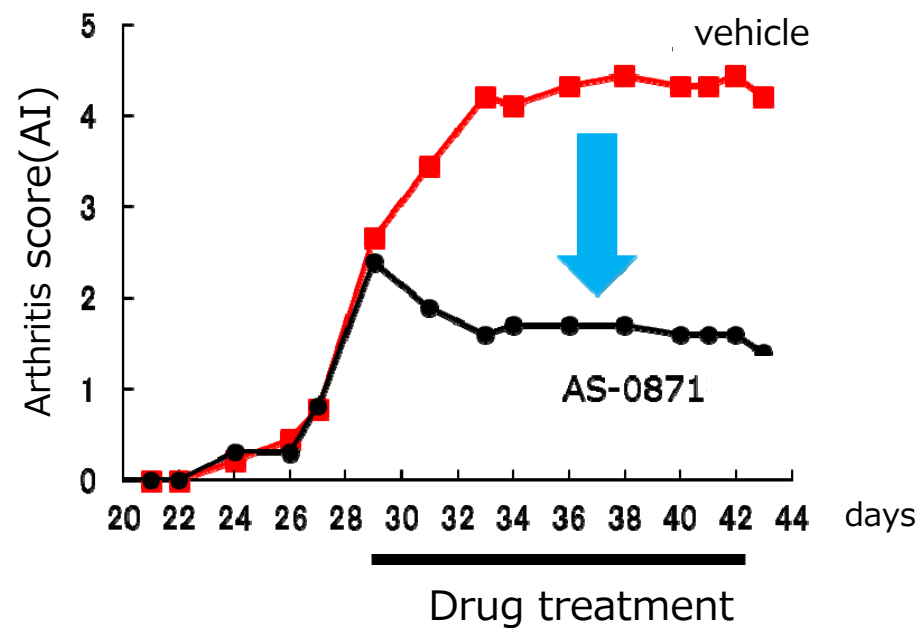


Vehicle



AS-0871

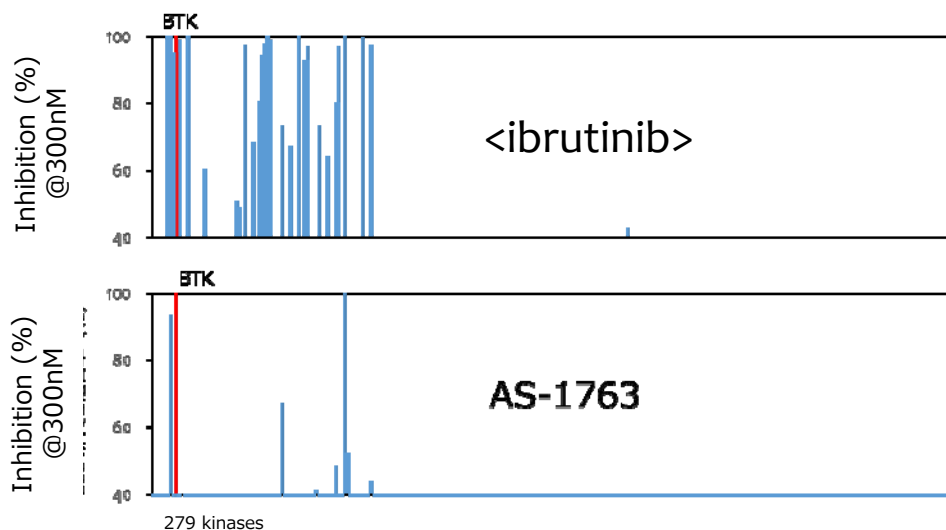
## Therapeutic efficacy in Collagen-induced arthritis (CIA) mice



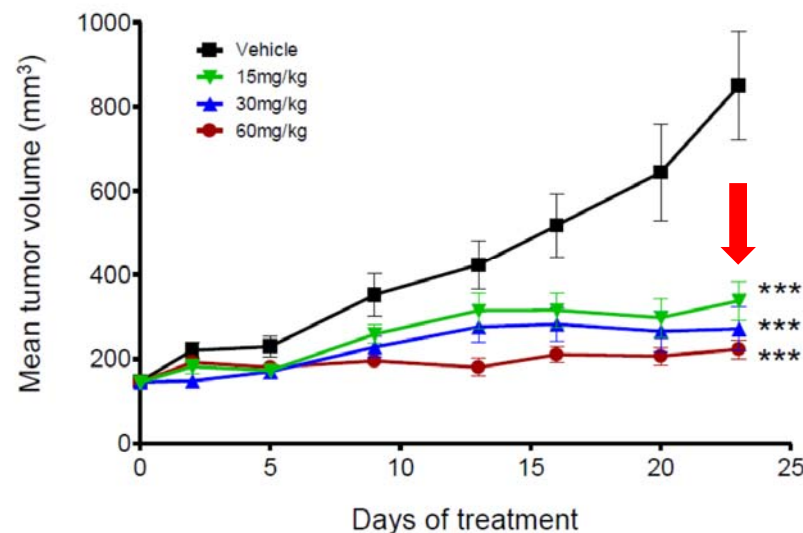
## AS-1763 : Development for Blood Cancer

- Small molecule BTK inhibitor
  - Non-covalent/reversible
  - High kinase selectivity
  - Inhibits both BTK wild type and ibrutinib resistant BTK C481S mutants
  - Orally available
  - Displayed strong anti-tumor effects in lymphoma model with both wild type and C481S mutant BTK.
  - Displayed efficacy in immuno-oncology model
  - Potential applications for autoimmune diseases
  - Plan to accelerate the clinical studies utilizing the clinical data of BioNova, the licensee in Greater China.
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- ✓ CTA (Clinical Trial Application) was approved by the Central Committee on Research Involving Human Subjects (CCMO) and the Ethics Committee in the Netherlands in February 2021.
  - ✓ Phase 1 single ascending dose (SAD) study in healthy volunteers was initiated in April 2021.
  - ✓ Plan to initiate Phase 1b study in patients in the U.S. after the completion of the SAD study. Documentation for an IND (Investigational New Drug Application) is undergoing.

## ◆ High kinase selectivity

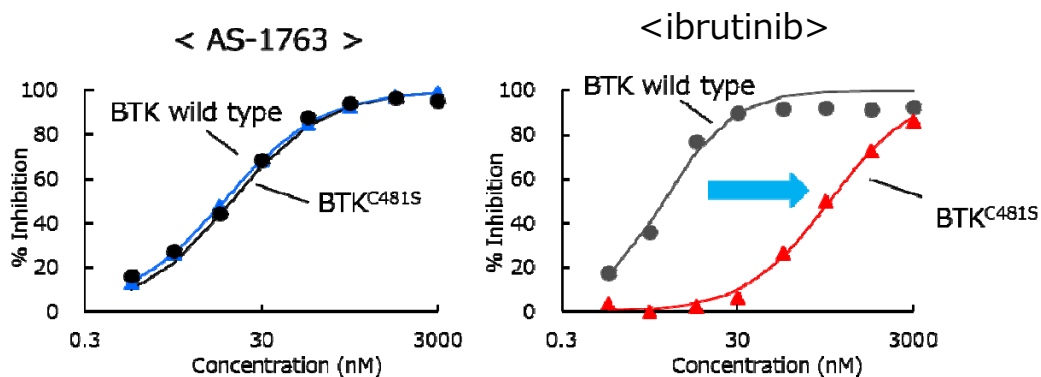


## ◆ AS-1763 significantly inhibits tumor growth in a B-cell lymphoma mouse model

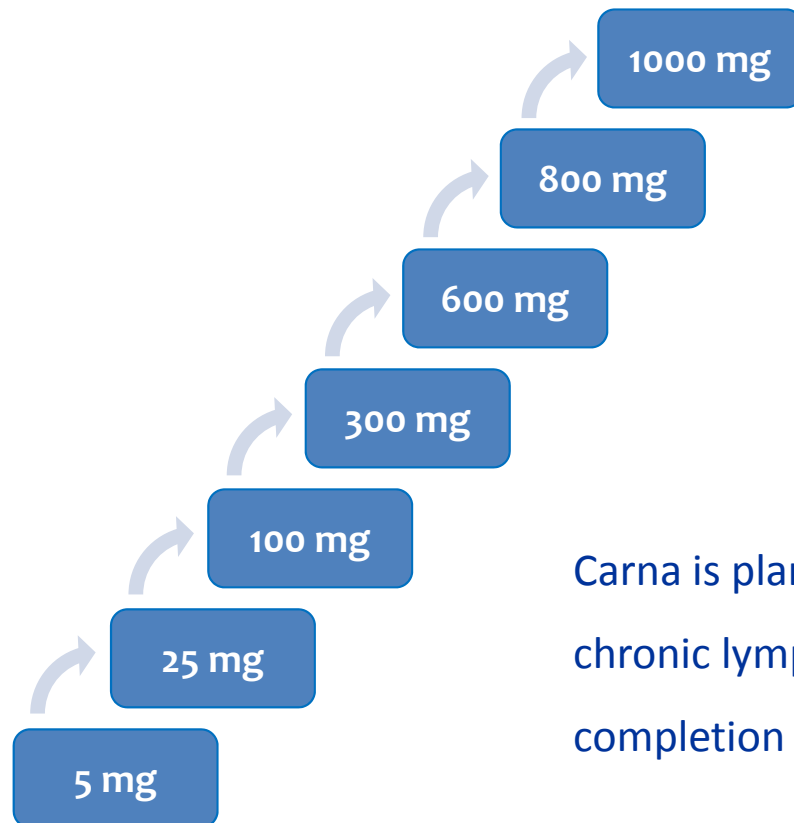


\*\*\* P<0.0001

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



## Single Ascending Dose Study(SAD)



- Placebo controlled (6 active / 2 placebo)
- Plan to perform 7 dose levels (8 subjects/cohort)
- Safety and tolerability
- Pharmacokinetics and pharmacodynamics

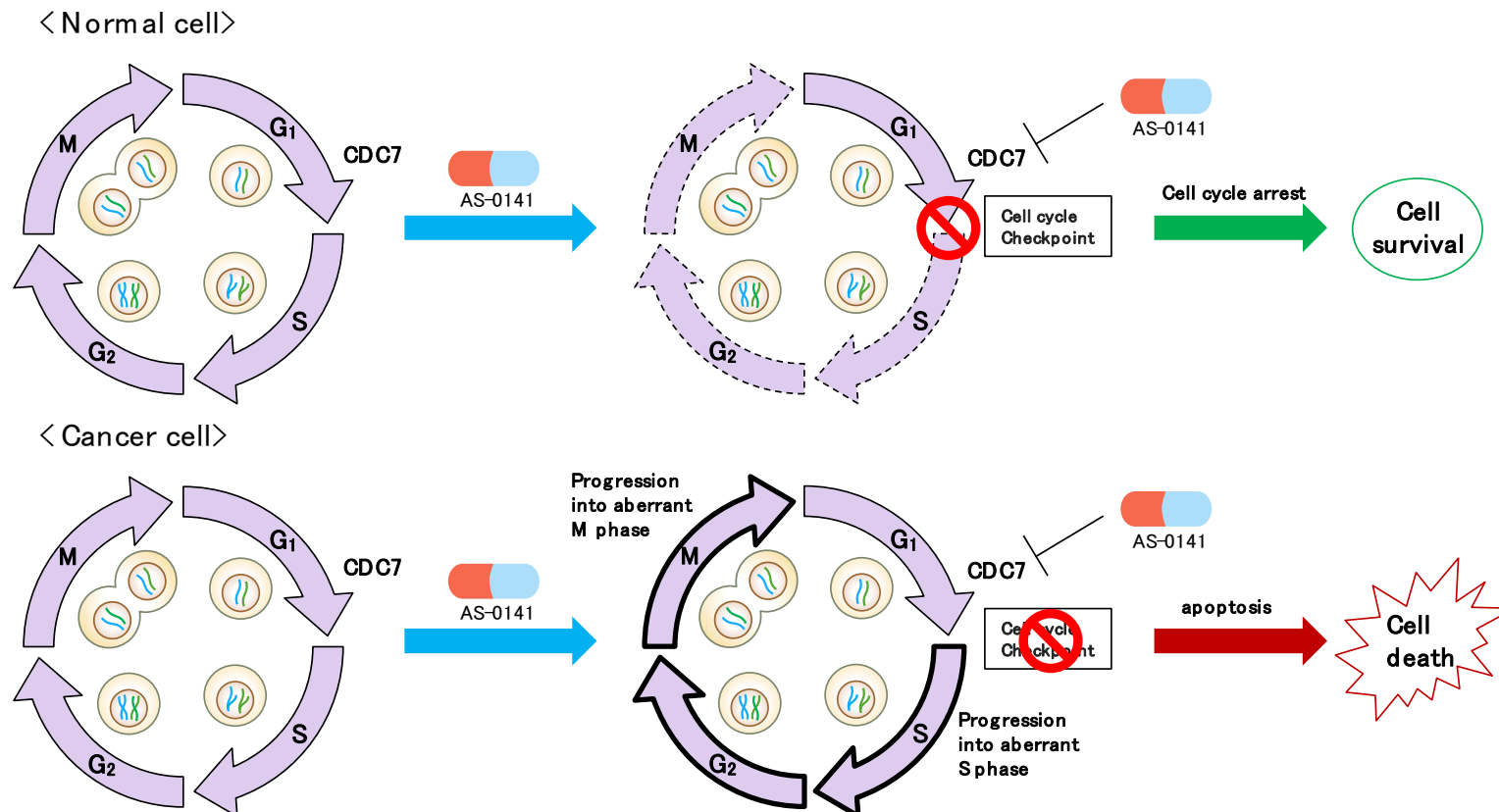
Carna is planning to conduct a Phase 1b clinical study in patients with chronic lymphocytic leukemia (CLL) and B cell malignancies following the completion of the SAD study.

## AS-0141 : Development for Cancer

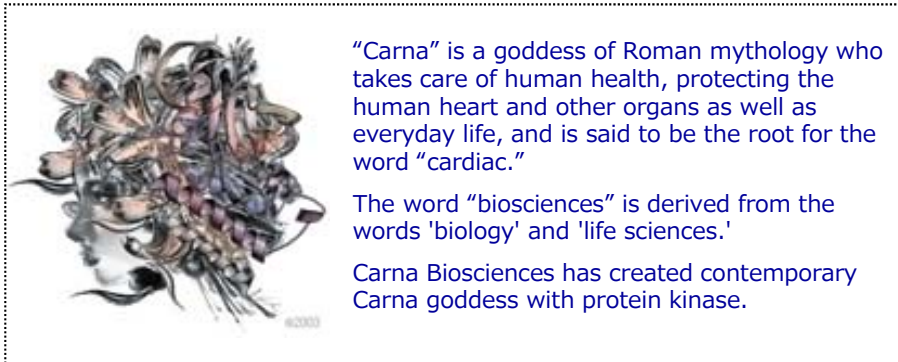
- |  |   |
|--|---|
| <ul style="list-style-type: none"><li>● Small molecule CDC7 inhibitor</li><li>● High kinase selectivity</li><li>● Potential First-in-class drug</li><li>● Orally available</li></ul> | <ul style="list-style-type: none"><li>● Potent anti-proliferative activity against various cancer cell lines</li><li>● Demonstrated strong anti-tumor activity in several human tumor xenograft models</li><li>● Planning a clinical study in Japan (1H 2021)</li></ul> |
|--|---|
- ✓ The review by the Pharmaceuticals and Medical Devices Agency (PMDA) for the clinical trial notification of AS-0141 to initiate phase I study in solid tumors was successfully completed in February.
  - ✓ Preparation undergoing to initiate a clinical study in Japan in H1 2021.
  - ✓ The Phase 1 clinical trial of AS-0141 is designed to assess the safety and tolerability of AS-0141 in advanced solid tumors, as well as to identify the recommended Phase 2 dose.

## ■ CDC7 kinase inhibitor

CDC7 (cell division cycle 7) is a serine-threonine kinase that plays a critical role in DNA synthesis and is required for the activation of DNA replication origins throughout the S phase of the cell cycle. Inhibition of CDC7 in cancer cells causes lethal S phase or M phase progression, whereas normal cells survive, most likely through induction of cell cycle arrest at the DNA replication checkpoint. It has been reported in the literature that CDC7 is overexpressed in many cancers. Therefore, CDC7 is an attractive target for cancer drug development.



- Sales at the Drug Discovery Support business in Q1 was JPY231 million, down 18.0% yoy.
  - ✓ In North America, sales to Gilead contributed.
  - ✓ Sales were on track in all areas to achieve full year plan.
- Robust start for the new service
  - ✓ Preparing to launch a full-panel assay service (192 kinds of kinases) for cell-based assay service using NanoBRET™ technology developed by Promega.
- Expanding lineup of kinase proteins and profiling service
  - ✓ 6 products, including high-demand mutant kinase biotinylated kinases, have been newly added to the line-up.
  - ✓ Proposing project-based service to collaborate with clients, leveraging Carna's drug discovery technology.



“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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