

# Financial Results FY2020 Q3

(January to September 2020)

Carna Biosciences, Inc.



Stock Code: 4572

#### FY2020 Q3 Key Highlights



#### <Drug Discovery>

- Initiation of our first FIH (First-in-Human) study.
- Initiated dosing in a FIH Phase 1 study of BTK inhibitor AS-0871 in August.
  - Continue to maximize the corporate value as a clinical-stage company

#### <Drug Discovery Support>

- Q1-Q3 sales are on track to achieve FY2020 forecast (76% vs. full year plan).
- Minimized the negative impact of COVID-19 with various measures.
- ✓ During the State of Emergency, employees were encouraged to work from home as much as possible to reduce interpersonal contacts. Drug discovery support team continued providing products and services as usual while taking measures such as shift work.
- ✓ We are currently operating as usual while taking various measures to prevent infection.



### FY2020 Q3 Results

#### FY2020 Q3

#### **Consolidated Financial Results**



(JPY mn)	FY2019 Q3 Actual	FY2020 Q3 Actual	YoY Change	FY2020 Plan	
Sales	2,862	847	-2,014 -70.4%	1,036	-Support business was strong in the U.SReceived an upfront payment in Q1 from licensingReceived an upfront payment from Gilead in Q2 2019.
Operating Profit/Loss	1,357	(615)	-1,972	(1,779)	-Investment in R&DUpfront payment decreased from the previous year.
Ordinary Profit/Loss	1,346	(625)	-1,971	(1,794)	
Net Profit/ Loss	1,113	(649)	-1,762	(1,822)	
R&D Cost	813	941	+128 +15.8%	2,040	-Investment in preclinical and clinical 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: FY2020 plan was disclosed on February 7, 2020.

# FY2020 Q3 Results by Business Segment



(JPY mn)	FY2019 Q3 Actual	FY2020 Q3 Actual	YoY Change	FY2020 Plan	vs. FY Plan	
Total Sales	2,862	847	-2,014 -70.4%	1,036	81.8%	
Drug Discovery Support	734	794	+60 +8.2%	1,036	76.7%	Sales in the U.S. were strong.
Drug Discovery & Development	2,128	53	-2,074 -97.5%		_	Received an upfront payment of JPY53 mn in Q1, compared to an upfront payment of JPY2.1b in Q2 2019.
Total Operating Loss	1,357	(615)	-1,972	(1,779)	_	
Drug Discovery Support	257	347	+90 35.2%	375	92.5%	Gross profit increased thanks to upbeat sales of internally developed products/services.
Drug Discovery & Development	1,099	(963)	-2,063	(2,155)	_	Investment in preclinical and clinical studies.

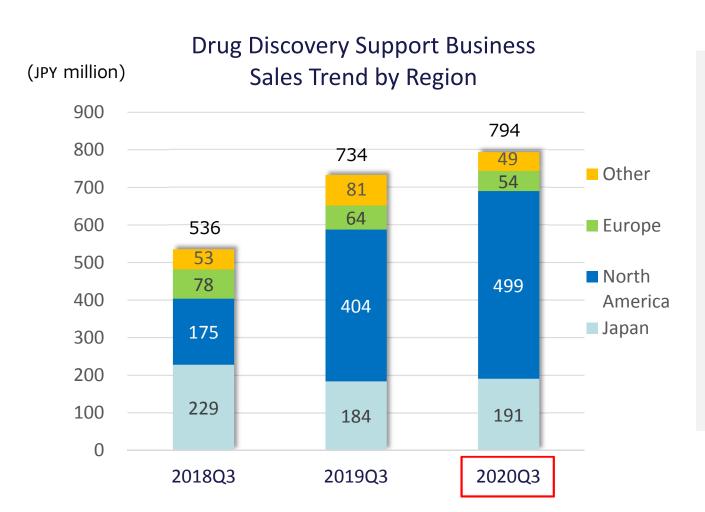
Note 1: Rounded down to the nearest million yen.

Note 2: YoY change % and comparison to FY2020 plan for Operating Loss are not presented since losses were recorded.

Note 3: FY2020 plan was disclosed on February 7, 2020.

# FY2020 Q3 Sales Trend by Region Drug Discovery Support Business





- Japan: Increased 3.6% YoY Profiling service and NanoBRET assay service were robust.
- North America: Increased 23.6% YoY Sales to Gilead and new biotech companies contributed positively.
- Europe: Decreased 15.3% YoY
  Kinase Proteins and assay development
  were weak.
- Other: Decreased 39.0%YoY Sales in China were weak compared to the strong performance in the previous year.

#### **Consolidated Balance Sheet**



(JPY mn)

			_	(JFT IIIII)
	As of Dec. 31, 2019	As of Sep. 30, 2020	Change	Reason for changes
Current assets	5,274	5,113	-161	
Cash and deposits	4,915	4,749	-165	
Non-current Assets	101	114	+12	
Total assets	5,376	5,227	-148	
Current liabilities	1,055	624	-430	Income taxes payable -120, Accounts payable -126
Non-current liabilities	467	313	-154	Long term borrowings -126 Bonds payable -28
Total liabilities	1,523	938	-585	
Total net assets	3,853	4,289	+436	Increase in share capital and capital surplus from exercise of share acquisition rights + 1,097, Net loss -649
Total liabilities and net assets	5,376	5,227	-148	
Shareholders' equity ratio	71.5%	82.0%		

 Share price of Carna
 71.5%
 82.0%

 82.0%
 329.8 yen
 345.6 yen

 988
 6.4 x
 4.3 x

 1,500 yen
 345.6 yen

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

#### **Financing**



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

- Funds raised from warrants are 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 Share Acquisition Rights

Series	Status of Exercise (as of the end of October)		
Series 18th Share Acquisition Rights	Total number of shares (73.5% of total warra Funds raised	s issued 1,195,000 shares ants issued) JPY2,173 million	

✓ With JPY4,749 million in cash and cash equivalents, we have sufficient funds to advance our R&D as planned.



### **Drug Discovery and Development**

### **Robust Pipeline**



#### <Oncology>

Compound	Target	Indication	Discovery/ Preclinical	Clinical	Partner
AS-0141	CDC7/ASK	Cancer			
Small Molecule	Kinase	Immuno-Oncology			<b>GILEAD</b>
AS-1763	ВТК	Blood Cancer Immuno-Oncology			Bioflova  Pharmaceuticals  **Greater China only
Small Molecule	Wnt-signal	Cancer Immuno-Oncology			
Small Molecule	TGFβ signaling	Blood Cancer Immuno-Oncology			
Small Molecule	CDK1	Cancer			

#### <Other Therapeutic Areas>

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

<sup>\*</sup> Other research projects are in the discovery phase to built next-generation pipeline.

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#### **AS-0141: Development for Cancer**

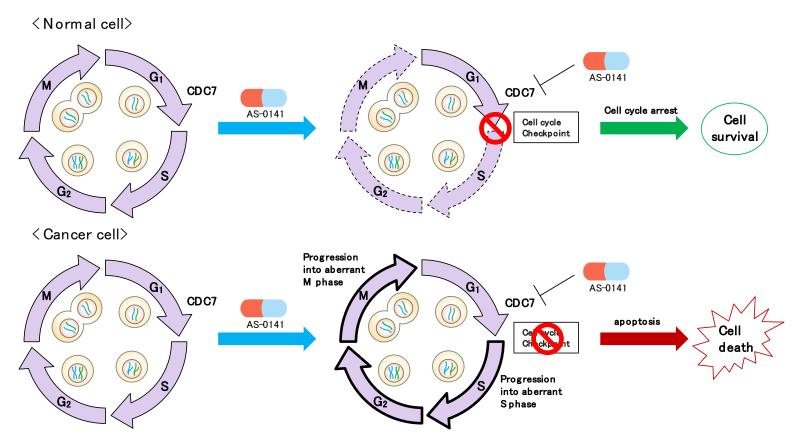
- Small molecule CDC7 inhibitor
- High kinase selectivity
- Potential First-in-class drug
- Potent anti-proliferative activity against various cancer cell lines
- Demonstrated strong anti-tumor activity in several human tumor xenograft models
- IND completed in the U.S.
- Planning a clinical study in Japan (1H 2021)
- ✓ Carna reacquired worldwide rights to AS-0141, licensed to Sierra Oncology in 2016, following Sierra's corporate prioritization of its portfolio to focus resource on the advancement of Phase 3 trials of momelotinib.
- ✓ IND package including all preclinical data and the API produced by Sierra have been transferred to Carna.
- ✓ Preparing for a clinical study in Japan, considering the risk of COVID-19 spreading in the U.S.
- ✓ Carna is planning a new clinical development strategy based on the scientific evidence to increase the probability of success, carefully analyzing the clinical data for the competitors' CDC7 inhibitors.

#### AS-0141: Highly Selective CDC7 Inhibitor



#### 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.





#### **AS-0871: Development for <u>Autoimmune Diseases</u>**

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

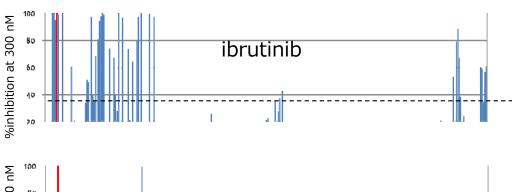
- Demonstrated significant efficacies in arthritis models
- Showed efficacy in systemic lupus erythematosus model
- Initiated Phase 1 study in August 2020
- ✓ The FIH study of AS-0871 was initiated on August 25 in the Netherlands, after the delay due to the COVID-19 pandemic in Europe.
- ✓ To date, AS-0871 is well-tolerated without any safety concerns, and continuing dose-escalation.
- ✓ The study is being carefully conducted with every measures taken against the COVID-19 at the clinical site.

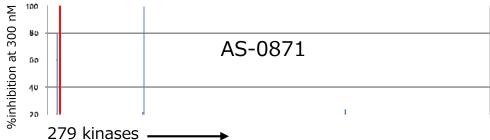
Step 1 Single Ascending Dose Study (SAD)	Step 2	
<ul> <li>Plan to perform 7 dose levels (8 subjects/cohort)</li> <li>Placebo controlled (6 active / 2 placebo)</li> <li>Safety and tolerability</li> <li>Pharmacokinetics and pharmacodynamics</li> </ul>	Food effect	Multiple Ascending Dose Study (MAD)

#### AS-0871: Highly Selective, Potent BTK inhibitor



#### ◆ High kinase selectivity





#### ◆ AS-0871 inhibits an allergic reaction

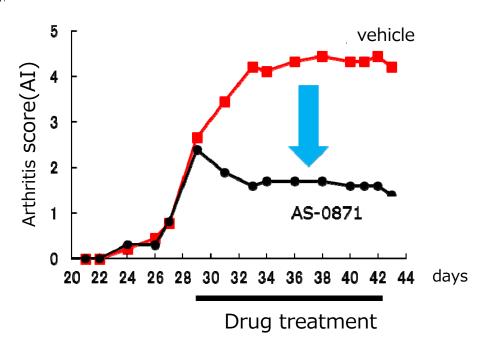




Vehicle

AS-0871

#### Therapeutic efficacy in Collageninduced arthritis (CIA) mice



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



#### **AS-1763**: Development for <u>Blood Cancer</u>

- 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
- Displayed efficacy in immuno-oncology model
- Potential applications for autoimmune diseases
- CTA submission targeted in 2020
- ✓ GLP toxicology studies required to initiate clinical study have been completed.
- ✓ Manufacturing the clinical trial drug product.
- ✓ Documentation for a CTA (Clinical Trial Application) is undergoing with a submission target by the end of 2020.
- ✓ We granted BioNova Pharmaceuticals an exclusive license to develop and commercialize AS-1763 in Greater China to conduct the clinical studies of AS-1763 in China, facilitating enrollment of potential patients.

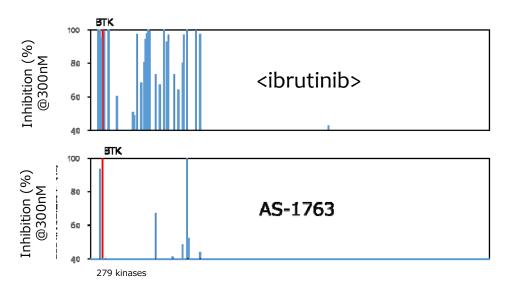
CTA: Clinical Trial Application in Europe

GLP: Good Laboratory Practice
GMP: Good Manufacturing Practice

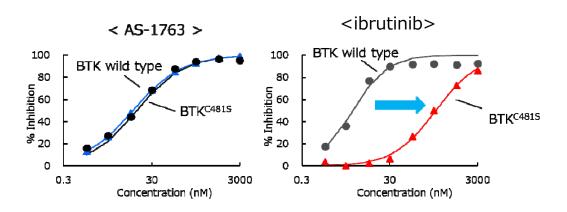
#### AS-1763: Strong Efficacy against C481S BTK mutant



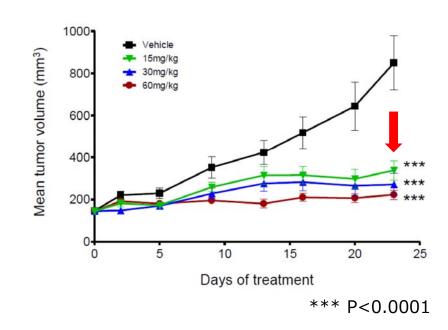
High kinase selectivity



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



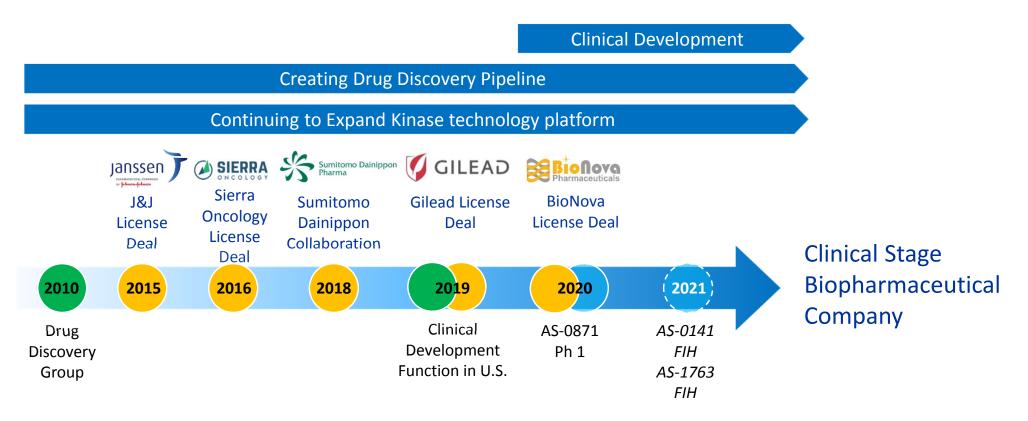
◆ AS-1763 significantly inhibits tumor growth in a B-cell lymphoma mouse model



#### **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 clinical development capabilities to conduct multiple clinical studies

FIH: First-in-Human

#### Our Research and Development Capabilities



#### **Drug Discovery**

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







#### Collaboration with Japanese Academia



State-of-the art science from Japanese Academia



Carna's drug discovery capability

- Drug discovery seeds
- Drug discovery technology
- Unmet medical needs
- Disease Mechanisms
- Biomarkers



#### Proven track record in drug discovery



Carna has signed partnerships with leading pharmaceutical companies including, Johnson & Johnson, Sierra Oncology, Sumitomo Dainippon Pharma, Gilead Sciences and BioNova Pharmaceuticals.













### **Drug Discovery Support**

#### Discovery Support Q3 FY2020 Key Highlights



- Drug Discovery Support business achieved sales of JPY794 million, up 8.2% yoy.
  - ✓ North America: +23.6% yoy thanks to sales to Gilead and biotech startups. Robust performance despite the slowdown in the customers' R&D activities due to the COVID-19 pandemic.
  - ✓ Japan: +3.6% yoy. Profiling service and cell-based assay service using NanoBRET<sup>TM</sup> technology were robust. The impact of the COVID-19 pandemic on sales has been small.
  - ✓ Europe: -15.3% yoy. Despite the impact of the pandemic, sales remained almost unchanged from the previous year until June thanks to a new order from a mega pharma. In Q3(Jul-Sep), sales decreased yoy compared to robust Q3 2019.
  - ✓ Other regions: -39.0% yoy. Activities of CROs in China seems to be slow due the pandemic in the U.S. and Europe.

### Discovery Support Measures to achieve FY2020 sales forecast



- We aim to achieve full-year sales forecast in the difficult environment where the spread of COVID-19 continues, especially in the U.S.
- While sales promotion by direct contact with customers are still restricted, we are identifying customer needs by frequent contacts using emails, phones, and web meetings, etc.
- We will continue developing new products such as biotinylated kinases, mutant kinases, or customized kinases to meet customer needs.
- Cell-based assay service using NanoBRET<sup>TM</sup>, licensed from Promega, has been growing steadily and we are taking various measures to expand sales further.





"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.

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