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CSTONE
PHARMACEUTICALS

CSTONE PHARMACEUTICALS (2616.HK)

2019 ANNUAL RESULTS PRESENTATION

March 27, 2020

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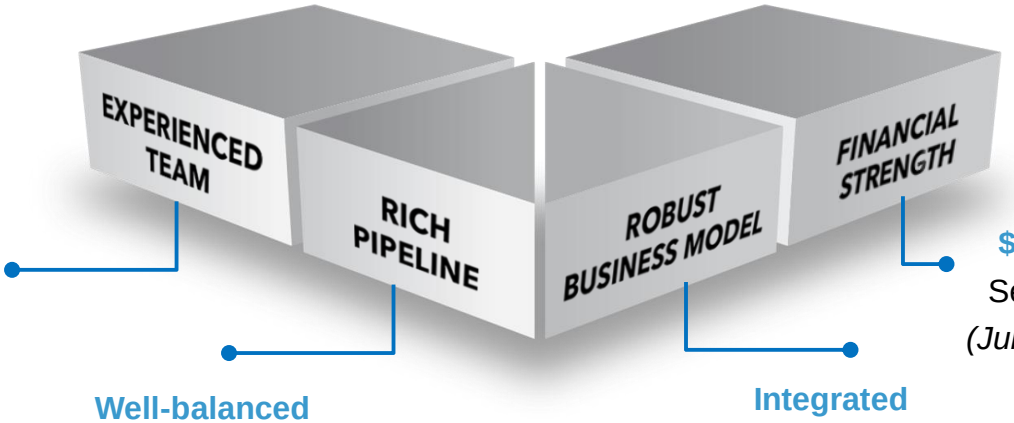
To Become **Globally** Recognized as the **Leading Chinese** Biopharma

4 Years Since Company Inception

HKEx listed
2616.HK



Industry-leading
Management Team

Well-balanced
Oncology Portfolio with a Focus on Immuno-oncology and Precision Medicine

Integrated
Biopharma with Clear Focus on Clinical Development

$150M Series A *(July 2016)* + **$262M** Series B *(May 2018)* + **$328M** HK IPO *(Feb 2019)*

Industry leading management team with proven track record and complementary expertise

Frank Jiang, MD, PhD
Chairman, Chief Executive Officer

SANOFI Lilly

Shirley Zhao, MD, MBA
Greater China GM,
Head of Commercial

Bristol-Myers Squibb Lilly Allergan SANOFI GENZYME

Jason Yang, MD, PhD
Chief Medical Officer

BeiGene COVANCE Pfizer AMGEN

Richard Yeh, MBA
Chief Financial Officer

Goldman Sachs citi AMGEN

Bing Yuan, PhD, MBA
Chief Strategy and
Business Officer

MERCK NOVARTIS Eisai

Jon Wang, PhD
Chief Scientific Officer

MERCK Biogen MedImmune

Archie Tse, MD, PhD
Chief Translational
Medicine Officer

MERCK Daiichi-Sankyo

Jingrong Li, PhD
SVP, Product Development
& Manufacturing

先声药业 Simcere BioSpecifics Roche

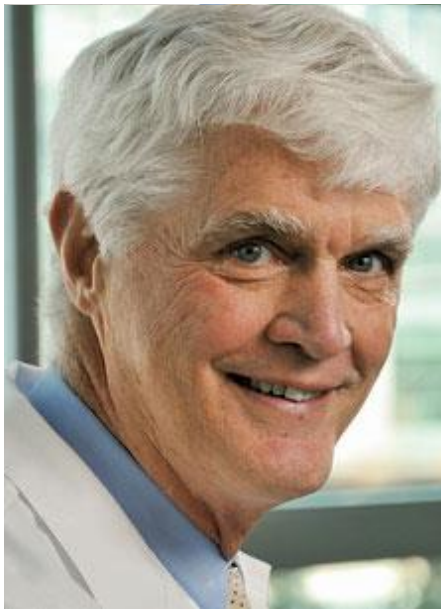
Sanhu Wang, MD
SVP, Government Affairs

饿了么 mindray 迈瑞
国家药品监督管理局 National Medical Products Administration

Yinghua Zhang
VP, Operations

先声默沙东 SMSD
先声药业 Simcere

Distinguished world-class scientific advisory board with deep oncology and IO expertise



Paul Bunn
MD

Former ASCO President
2002-2003
Distinguished Professor,
University of Colorado

Elizabeth Jaffee
MD

Former AACR President
2018-2019
Professor of Oncology,
Johns Hopkins
University

Weiping Zou
MD, PhD

Chair, AACR Cancer
Immunology
Charles B.de Nancrede
Professor,
University of Michigan

Richard Finn
MD

Former International
Liver Cancer
Association President
Clinical Professor,
UCLA

Note: ASCO = American Society of Clinical Oncology; AACR = American Association for Cancer Research.

Well-balanced oncology portfolio with a focus on immuno-oncology and precision medicine

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	Drug Candidate	Lead Indication(s) and Line(s) of Therapies	Rights	Pre-clinical	Dose Escalation	POC	Pivotal	NDA	Partner
Late-stage	CS1001 (PD-L1)	R/R cHL, R/R NKTL, NSCLC, GC, EC	Global						
	CS1003 (PD-1)	HCC, Solid tumors	Global						
	Ivosidenib (IDH1)	R/R AML, 1L AML, Cholangiocarcinoma	China					Taiwan NDA submitted	agios
	Avapritinib (KIT / PDGFRA)	PDGFRA / 3L GIST, AdvSM, ISM	China						blueprint MEDICINES
	Pralsetinib (RET)	1L / 2L NSCLC, 1L MTC[4]	China						blueprint MEDICINES
Clinical/IND	Fisogatinib (FGFR4)	1L / 2L HCC	China						blueprint MEDICINES
	CS1002 (CTLA-4)	Solid tumors	Global						
	CS3006 (MEK)	Solid tumors	Global						
	CS3003 (HDAC6)	Solid tumors, R/R MM	Global						
	CS3002 (CDK4/6)	Solid tumors	Global						
	CS3005 (A2aR)	Solid tumors	Global						
Pre-clinical	NM21-1480 (PD-L1/4-1BB/HSA)	Undisclosed	Korea, China, Singapore						NUMAB
	CS1009		Global						
	CS3004		Global						
	CS2004		Global						

Global China Korea Singapore

Source: Company

Note: Assets status denote progress in the region noted in the column titled "Rights". AML= Acute Myeloid Leukemia, AdvSM = Advanced Systemic Mastocytosis, cHL= Classical Hodgkin's Lymphoma, GIST = Gastrointestinal Stromal Tumor, HCC = Hepatocellular Carcinoma, ISM = Indolent Systemic Mastocytosis, NKTL = Natural KILLER/T Cell Lymphoma, NSCLC = Non-small Cell Lung Cancer, MTC = Medullary Thyroid Cancer, R/R = Relapsed or Refractory, SM = Systemic Mastocytosis, MM = Multiple Myeloma.

Focus on China's largest indications, covering 55%+ of total cancer incidences

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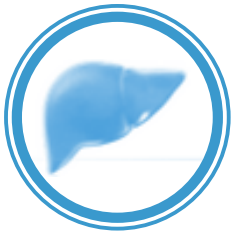


Cancer type	Lung Cancer	Colorectal Cancer	Gastric Cancer	Liver Cancer	Esophagus Cancer
New cases (2018)	774,323	516,859	456,124	392,868	307,359
5-year prevalence (2018)	716,411	1,237,145	603,851	296,780	284,163
Ongoing and planned clinical trials	PD-L1 Mono Stage III NSCLC	PD-L1+Regorafenib Advanced CRC	PD-L1 + Chemo Advanced GC/GEJ	PD-1 + VEGFRi Advanced HCC	PD-L1 + Chemo Advanced ESCC
	PD-L1 + Chemo Stage IV NSCLC	PD-1+Regorafenib Advanced CRC	PD-L1+Regorafenib Advanced GC/GEJ	Fisogatinib FGF19+ HCC	
	Pralsetinib RETm 1L NSCLC & 2L NSCLC		PD-1+Regorafenib Advanced GC/GEJ	PD-L1 + Fisogatinib FGF19+ HCC	

registrational
exploratory

Source: Globocan 2018, represents estimated data in China
Note: NSCLC = non-small cell lung cancer; GC = gastric adenocarcinoma; GEJ = gastro-esophageal junction adenocarcinoma; HCC = hepatocellular carcinoma; ESCC = esophageal squamous cell carcinoma; CRC = colorectal cancer; VEGFRi = inhibitor of vascular endothelial growth factor receptor

Sizable portfolio anchored around 3 IO backbone agents to drive differentiated combo strategy

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3 IO backbone agents

- Only company in China owns clinical stage ***PD-L1, PD-1 and CTLA-4***

15 assets in the pipeline

- ***10*** in-house developed de-risked assets plus ***5*** in-licensed FIC/BIC assets

1 potential 2nd generation of PD-(L)1

- ***PD-L1x4-1BB*** *provides more flexible* combo and potential better efficacy

De-risked Combo

- PD-L1 + chemo/radiation
- PD-(L)1 + Regorafenib
- PD-1 + CS1002 (CTLA-4)
- PD-L1 + Donafenib

Novel Combo Unique to CStone

- PD-L1 + Fisogatinib (FGFR4)
- PD-(L)1 + CS3002 (CDK4/6)
- PD-1 + Ivosidenib (IDH1)
- Potentially more…

Multi-specific/Multi-functional

- NM21-1480 (PD-L1 x 4-1BB)
- Potentially more…

11 combo trials, including **5** registrational trials

Clinical Development Engine: Strong in-house team led by experienced leaders and supported by global CROs

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Frank Jiang, MD, PhD
Chairman and CEO

- Former Head of APAC R&D for Sanofi
- Led a **21,000** patient mega-trial
- Led **79** clinical trials and **30** NDAs within five years

Jason Yang, MD, PhD
CMO

- Former SVP and Head of Clin Dev for Beigene, led development of PD-1, BTK, PARP and RAF dimer inhibitors
- Led **40+** global and China trials

Archie Tse, MD, PhD
CTMO

- Former Executive Director of Early Clin Dev at MSD (US)
- Led **30+** FIH oncology trials
- Led **20+** I/O combination trials

Responsible for late stage clinical development and regulatory affairs

- Clinical Development & PV (1)
- Regulatory Affair
- Biometrics & Medical Writing
- Quality Assurance
- Clinical Operations

Responsible for early stage clinical development and diagnostics/biomarkers

- Molecular Diagnostics
- Translational Medicine & Biomarker
- Clinical Pharmacology
- Early Development

Strong in-house team with ~170 clinical staff, representing ~60% of total employees, of which ~75% hold advanced degrees (2) and ~80% have clinical development experience at MNCs

IMS Health & Quintiles are now IQVIA™ | PAREXEL® | COVANCE SOLUTIONS MADE REAL® | Syneos Health

Keep clinical strategy planning & development oversight in-house, while outsourcing day-to-day execution to global CROs to ensure optimal balance between efficiency and scalability

(1) *Includes GI cancer, lung cancer, hematology & other solid tumors, and pharmacovigilance*
(2) *Master and above*

Significant clinical progress - overview

28 trials including 5 assets in 13 registrational trials today

CS1001 (PD-L1)	■ **6** registrational trials in China ■ Stage III NSCLC, stage IV NSCLC, GC, ESCC, NKTL and cHL
CS1003 (PD-1)	■ **1** registrational trial ■ HCC (global)
Ivosidenib (IDH1)	■ **2** registrational trials ■ IDH1m AML, R/R AML
Avapritinib (KIT&PDGFRA)	■ **2** registrational trials ■ PDGFRA exon 18 GIST, 3L GIST
Pralsetinib (RET)	■ **2** registrational trials ■ 2L NSCLC, 1L MTC

Note: AML= Acute Myeloid Leukemia,, cHL= Classical Hodgkin's Lymphoma, GIST = Gastrointestinal Stromal Tumor, HCC = Hepatocellular Carcinoma, NKTL = Natural KILLER/T Cell Lymphoma, NSCLC = Non-small Cell Lung Cancer, EC = Esophagus Cancer, MTC = Medullary Thyroid Cancer, R/R = Relapsed or Refractory

Significant clinical progress – CS1001 (PD-L1) (1/5)

Asset overview

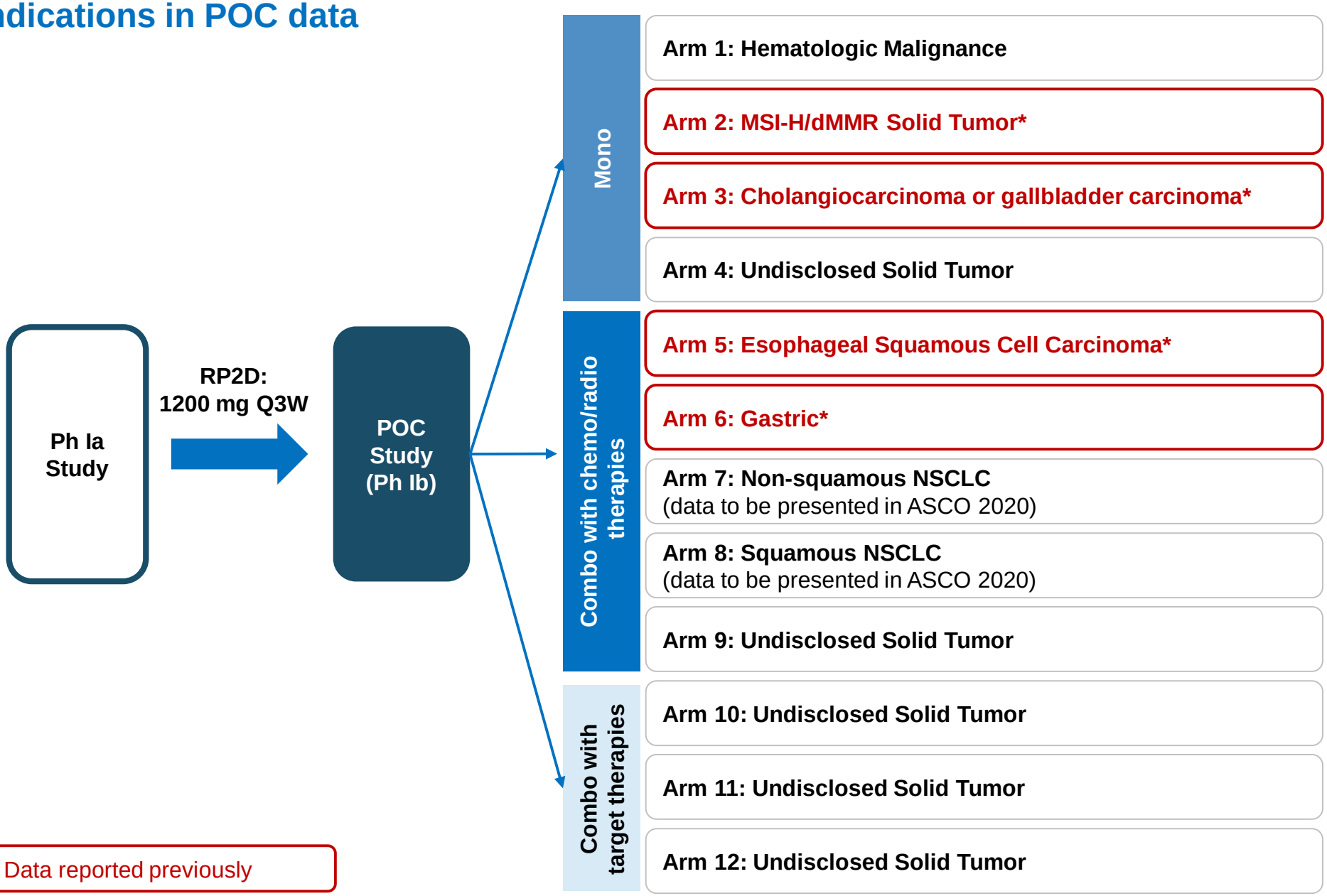
Unique design	■ **Fully-human, full length IgG4** derived from Ligand's OmniRat® platform – minimal possibility of generating ADA; ■ The only PD-L1 antibody that *naturally* lacks of ADCC/CDC activity – better safety and avoid unwanted attack of T cells; ■ Retains ADCP activity that potentially induces direct tumor killing by macrophages and enhances tumor antigen presentation for long-term anti-tumor immunity – more efficacious in certain indications like NKTL
Prominent safety profile	■ Early phase studies demonstrated that CS1001 was **safe & well tolerated** – No DLT[1] from 3 mg/kg to 40 mg/kg, MTD[2] not reached – No infusion reactions; low ADA[3] rate – Low frequency of severe irAEs
Best-in-class potential	■ POC data of CS1001 showed **potential of best-in-class PD-L1** – **Encouraging anti-tumor activities** observed in phase Ia dose-escalation study, multiple phase Ib cohorts and phase II studies – In particular, promising data in **ESCC** and in **NKTL**
Significant development progress	■ **>1,200** patients dosed, with **6 registration trials** ongoing, including 2 pivotal Ph II studies and 4 Ph III studies ■ Strategically targeting China prevalent cancers including S3 and S4 lung cancer, GC and EC

** as of Jan 2, 2020*

1. *DLT: dose limiting toxicity*
2. *MTD: maximum tolerated dose*
3. *ADA: anti-drug antibodies*

Significant clinical progress – CS1001 (PD-L1) (2/5)

Phase Ia & Ib study data: best-in-class potential in multiple indications in POC data



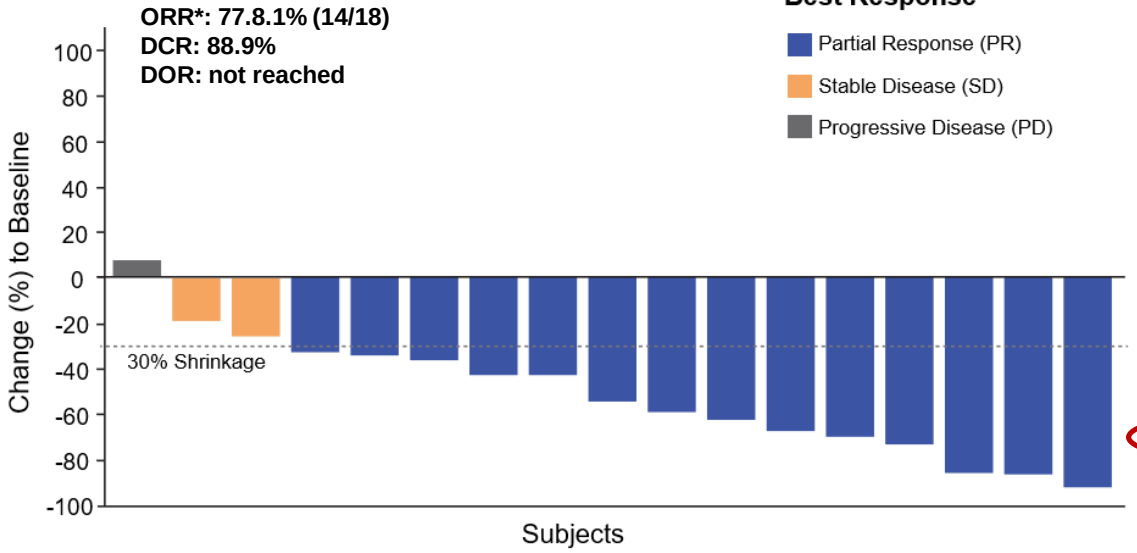
* Data reported previously

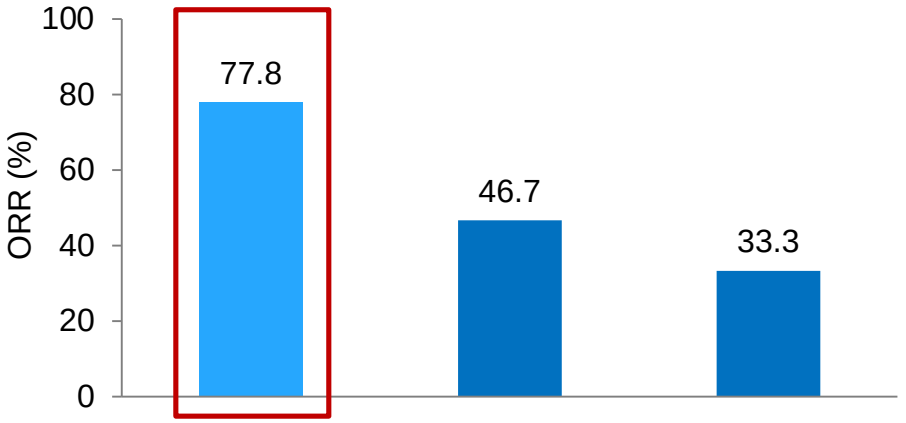
Significant clinical progress – CS1001 (PD-L1) (3/5)

Phase Ib study data: CS1001 + CF (Cisplatin + 5-FU) in 1L ESCC

Esophageal Squamous Cell Carcinoma:

ORR=77.8% (14/18), DCR=88.9% (16/18)





	CS1001	Tislelizumab	Durvalumab+ Tremelimumab
Class	PD-L1	PD-1	PD-L1
n	23	15	6
ECOG	**0: 21.3% 1: 78.3%**	**0: 26.7% 1: 73.3%**	**0: 0% 1: 100%**
Chemo Regimen	CF	CF	CF
DOR (m)	NR (0.03+~8.4+)	12.8	Not reported
Source	CSCO 2019	CSCO 2019	ASCO GI 2019

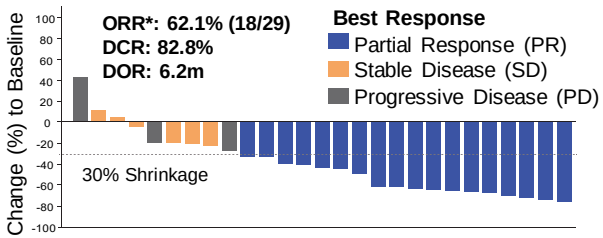
Source: Company, Ph1b data presented at 2019 CSCO
Note: ORR = Overall Response Rate; DOR = Duration Of Response;

Significant clinical progress – CS1001 (PD-L1) (4/5)

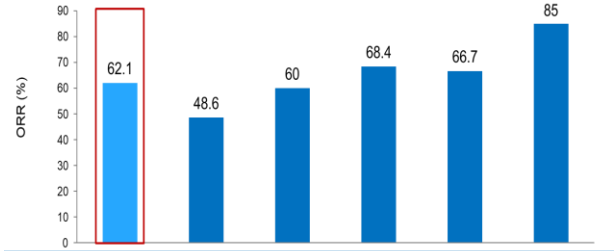
Phase Ib study data: other cohorts

CS1001 + XELOX (Capecitabine + Oxaliplatin) in 1L GC/GEJ (Gastric or Gastroesophageal Junction Adenocarcinoma)

Target Lesion Shrinkage from Baseline (N=29)



* 15 confirm PR; 3 PR to be confirmed as of data cut off on 1 July 2019

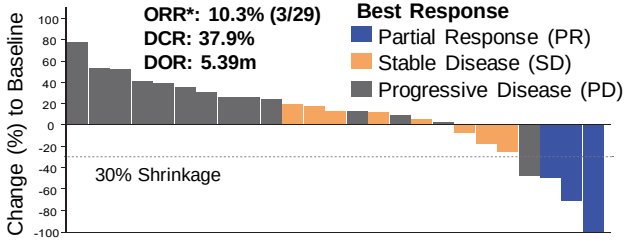


	CS1001	Keytruda (062)	Keytruda (059)	Opdivo	Camrelizumab	Sintilimab
Class	PD-L1	PD-1	PD-1	PD-1	PD-1	PD-1
n	21	257 vs 250*	25	38	48	20
ECOG	0: 41.4% 1: 58.6%	0: 46.0% 1: 54.0%	0: 60.0% 1: 40.0%	0: 50.0% 1: 50.0%	0: 41.7% 1: 58.3%	0: 45.0% 1: 55.0%
Chemo Regimen	XELOX	Cisplatin + 5-FU or Capecitabine	Cisplatin + 5-FU or Capecitabine	SOX or XELOX	XELOX	XELOX
DOR (m)	6.2 (0.03+ ~ 6.21+)	6.8	4.6 (2.6 ~ 20.3+)	9.9 (5.8, NR)	NR	5.3 (4.8~7.2)
Source	CSCO 2019	ASCO 2019	ESMO 2017	Ann Oncol. 2019 Feb 1;30(2):250-258	ASCO 2019	ASCO 2019

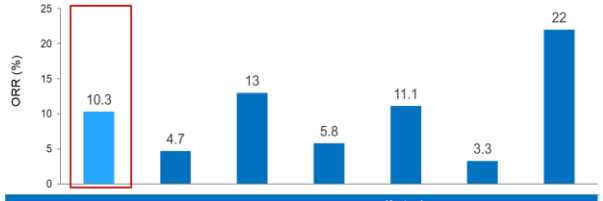
* ORR of Cisplatin + 5-FU or Capecitabie was 37.3%

CS1001 (PD-L1) as monotherapy in CC/GBC (Cholangiocarcinoma or Gallbladder Carcinoma)

Target Lesion Shrinkage from Baseline (N=29)



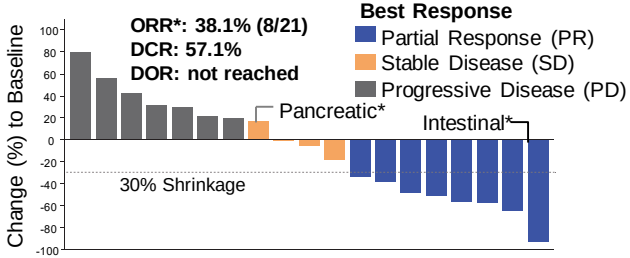
* 2 confirm PR; 1 PR to be unconfirmed as of data cut-off on 1 July 2019



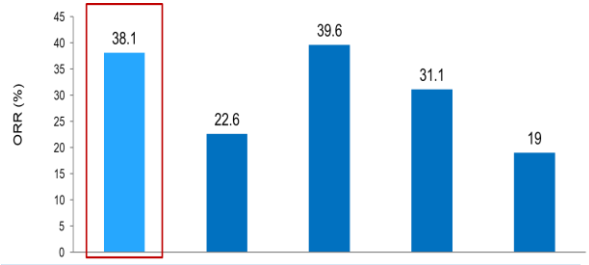
	CS1001	Imfinzi Asia	Keytruda (PD-L1>=1%)	Keytruda (global)	Keytruda (PD-L1>=1%) Korean	Opdivo Japan	Opdivo US
Class	PD-L1	PD-L1	PD-1	PD-1	PD-1	PD-1	PD-1
n	29	42	24	104	39	30	54
ECOG	0: 14(48.3) 1: 13(44.8) NA: 2 (6.9)	0: 27(64) 1: 15(36)	0: 9(37.5) 1: 15(62.5)	0: 42(40.4) 1: 62(59.6)	Not reported	Not reported	Not reported
Regimen	1200mg IV Q3W	10 mg/kg Q2W	10mg/kg IV Q2W	200mg IV Q3W	200mg IV Q3W	Mono: nivolumab 240mg Q2W	240mg Q2W for 16 weeks then 480mg Q4W
DOR (m)	5.39 (1.91+,8.02)	9.7	NR (21.5 -29.4+)	NR (6.2-23.2+)	Not reported	Not reported	Not reported
Source	CSCO 2019	JCO 2019	ASCO 2019	ASCO 2019	JCO 2019	Lancet 2019	JCO 2019

CS1001 (PD-L1) as monotherapy in MSI-H/dMMR Cancer

Target Lesion Shrinkage from Baseline (N=29)



** Unless specified, all patients were Colorectal Cancer



	CS1001	Imfinzi	Keytruda	Opdivo	Tislelizumab
Class	PD-L1	PD-L1	PD-1	PD-1	PD-1
n	21	62	149	74	16
ECOG	0: 0 1: 100%	Not reported	0:36% 1:64%	0:43% 1:57%	Not reported
Regimen	1200mg iv q3w	10mg/kg q2w	200mg q3w /10mg/kg q2w	3mg/kg q2w	200mg q3w
DOR (m)	NR (0.03+, 8.6+)	Not reported	NR (1.6+, 22.7+)	NR	NR
Source	CSCO 2019	ASCO GI 2019	PI, 05/2017	Lancet Oncol 2017	CSCO 2019

Significant clinical progress – CS1001 (PD-L1) (5/5)

Phase II study data: CS1001 as monotherapy in rr-ENKTL

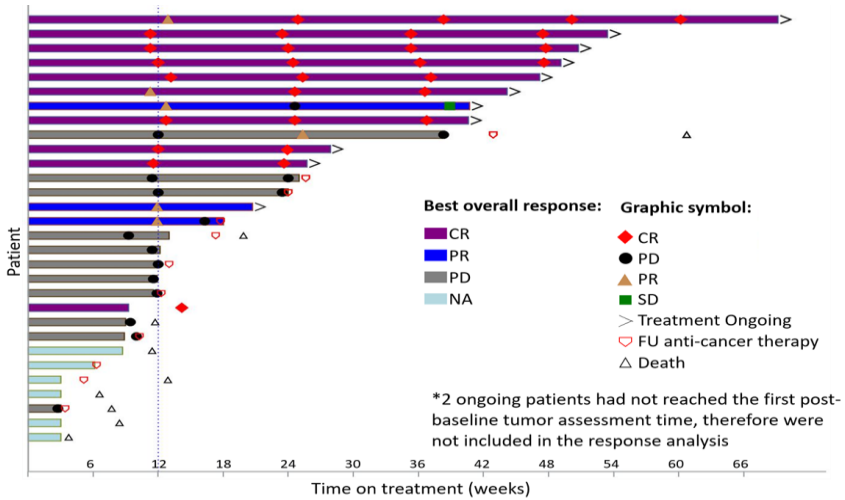
Preliminary efficacy data

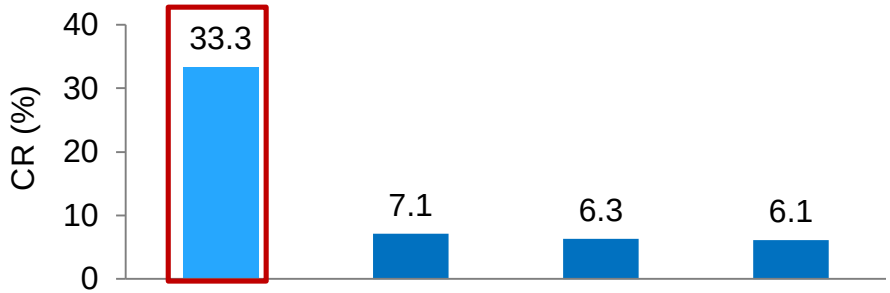
CS1001 demonstrated promising antitumor activity with a high CR rate and durable response in rr-ENKTL patients

- Among the 22 efficacy-evaluable patients, the investigator-assessed ORR was 40.9%
- 7 **(31.8%)** patients achieved complete response
- The duration of response (DOR) ranged from 0.03+ to 8.61+ months, and the median DOR was not reached
- 8 additional pts reached response assessment time point, the updated data was reported as poster presentation at the 2019 ASH conference

Duration of treatment, Best response, Duration of response (N=30)

ORR = 43.3% (13/30), CR = 33.3.% (10/30), DOR not reached
1Yr OS rate: 72.4% as of data cut-off on 8 Oct 2019





CR is more clinically meaningful for r/r ENKTL

- Patients with CR have long duration of response while patients with PR generally progress quickly [1,2].
- High CR rate translates to potential allogeneic HSCT and curation of the disease [3].

	CS1001	Sintilimab	Chidamide (Approved in rr-PTCL, China, 2014)	
Class	PD-L1	PD-1	HDAC	
Study	CS1001-201	ORIENT-4	Registration Study	Real World Study
n	30	28	16	33
CR	**33.3%** (10/30)	7.1% (2/28)	6.3% (1/16)	6.1% (2/33)
DOR (m)	NR (0.03+~8.61+)	4.1 (0+,4.2+)	UNK	UNK
Source	ASH 2019	ASCO 2019	Ann Oncol, 2015[4]	J Hematol Oncol, 2017[5]

Significant clinical progress – CS1003 (PD-1) and CS1002 (CTLA-4)

Two Additional I/O Backbones: preliminary data and development strategy

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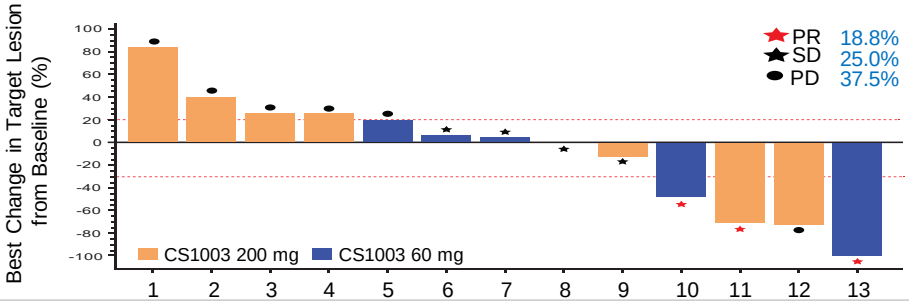
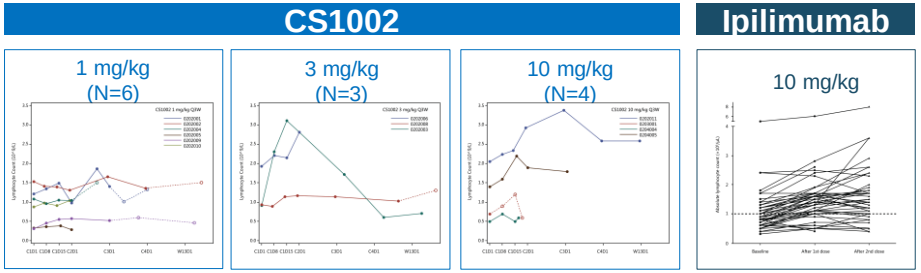
	CS1003	CS1002
Asset overview	■ Humanized IgG4 anti-PD-1 mAb ■ Recognize both human & murine PD-1 with unique advantage to evaluate efficacy in syngeneic mouse models, esp. for testing combinations with small molecules	■ A full-length, fully human IgG1 mAb against CTLA4 ■ Amino acid sequence identical to ipilimumab (Yervoy)
Ph1 Data Highlights	■ Bridging Ph1 conducted in China showed that CS1003 monotherapy was safe and tolerable at 60mg and 200mg Q3W; no DLT or MTD was observed (N=19) ■ Dose proportional increase in systemic exposure to CS1003 ■ Preliminary anti-tumor activity of CS1003 observed in multiple tumor types (PR 3/16 (18.8%))	■ CS1002 monotherapy was well tolerated up to 10 mg/kg Q3W, with no DLT and no MTD reached (N=13) ■ CS1002 demonstrated dose-proportional PK; increase in absolute lymphocyte count (ALC) observed indicating target engagement ■ Overall clinical profile is consistent with that of ipilimumab
Strategic value	■ One of 3 I/O backbones with current safety and efficacy data supporting further development ■ Initiated combo study with CS1002, and combo study with lenvatinib in Ph3 trial for 1L HCC	■ Potentially become another outstanding CTLA-4 inhibitor after Ipilimumab, which has not been launched in China ■ One of 3 I/O backbones to enable flexible combo strategy, including chemo-free I/O-I/O combo with CS1003

Preliminary efficacy in multiple tumor types



CS1002 induced early ALC increase, similarly to Ipilimumab



DLT: dose-limiting toxicity; MTD: maximum tolerated dose; Q3W: once every 3 weeks; PK: pharmacokinetics; ADA: anti-drug antibody; PR: partial response; VEGF: vascular endothelial growth factor; TKI: tyrosine kinase inhibitor; HCC: hepatocellular carcinoma

1=Squamous cell carcinoma of the cervix; 2=Synovial sarcoma; 3=Hepatocellular carcinoma; 4=Gastric adenocarcinoma; 5=Leiomyosarcoma; 6=Malignant melanoma; 7= Leiomyosarcoma; 8= Fibromatosis; 9= Follicular dendritic cell sarcoma; 10=Uterine Leiomyosarcoma; 11= Laryngeal squamous cell carcinoma; 12= Oesophageal squamous cell carcinoma; 13= Oesophageal squamous cell carcinoma.

Significant clinical progress – ivosidenib (CS3010)

Global and China development and regulatory status

Global

Partner	Drug Candidate	Target	Indications	Pivotal / Registrational Trial Patient Enrollment	NDA Submission	NDA Approval
agios	**Ivosidenib**	IDH1	R/R AML			✓
			IC-Ineligible 1L AML monotherapy			✓
			IC-Ineligible 1L AML combo with AZA	✓		
			2/3L Cholangiocarcinoma monotherapy	✓		

✓ Achieved

Greater China

Partner	Drug Candidate	Indications	Region	Mono /Combo	Study Initiation	Patient Enrollment	NDA Submission	NDA Approval
agios	**Ivosidenib (CS3010)**	IDH1m R/R AML	Taiwan	Mono	Registration with US NDA data		submitted	
		IC-Ineligible 1L AML combo with AZA	China	Combo	Joining global study			
		IDH1m R/R AML	China	Mono	Bridging study			

TIBSOVO®: global first-in-class IDH1 inhibitor

No immediate competitor in China; significant indication expansion potential

In Partnership With

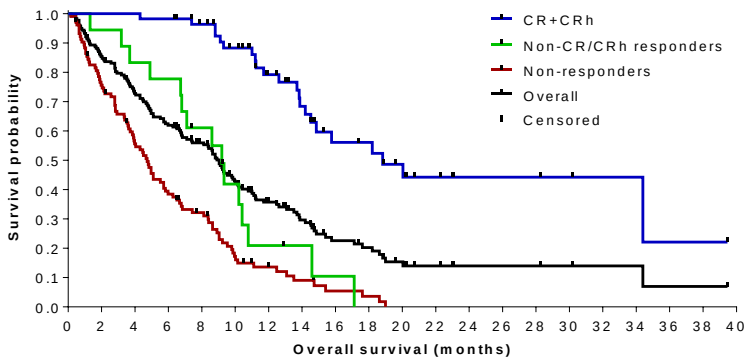
agios

China Competition Overview

First China NDA Filing	2021
First in Class molecule	✓
# of Competitor(s) in Clinical Development	1
Lead over immediate competitor	>3 years
Global Patent Expiry	2033

Durable Remissions with Ivosidenib in IDH1-Mutated Relapsed or Refractory AML

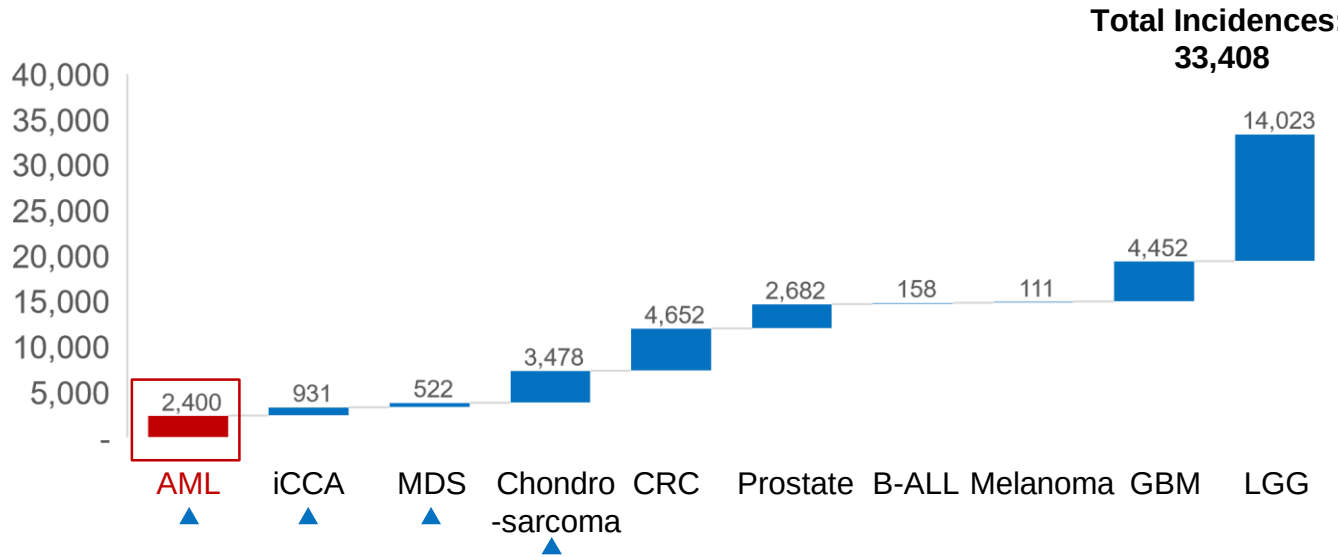
The NEW ENGLAND JOURNAL of MEDICINE



U.S. FOOD & DRUG ADMINISTRATION

- ✓ Fast Track Review
- ✓ 2X Breakthrough Therapy Designation
- ✓ Priority Review

Total Newly Diagnosed Addressable Incidences (2018)



Cancer Types Harbouring IDH1 Mutation

Source: Globocan 2018; CStone analysis

▲ *Active Clinical Programs*

Significant clinical progress – avapritinib (CS3007)

Global and China development and regulatory status

Global

Partner	Drug Candidate	Target	Indications	Pivotal / Registrational Trial Patient Target Enrollment	U.S. NDA Submission	U.S. NDA Approval
blueprint MEDICINES	Avapritinib (CS3007)	KIT & PDGFRA	PDGFRA exon 18 GIST			✓
			4L GIST		✓	
			3L GIST	✓	✓	
			Advanced SM	✓(1)	✓	

✓ Achieved ✓ To be expected in 2020

Greater China

Partner	Drug Candidate	Indications	Region	Mono /Combo	Study Initiation	Patient Enrollment	NDA Submission	NDA Approval
blueprint MEDICINES	Avapritinib (CS3007)	PDGFRA Exon 18	Taiwan	Mono	Registration with US NDA data		★	
		GIST 3L	China	Mono	Joining global study		★	
		PDGFRA Exon 18	China	Mono	Bridging study		★	
		4L GIST	China	Mono	Bridging study			
		Advanced SM	China	Mono	Exploring trial waiver			

Note: AYVAKIT™ (avapritinib) is approved by the U.S. FDA for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations

(1) Sufficient enrollment has been achieved to enable interim analysis supporting an H2 2020 filing, but the PATHFINDER trial is not fully enrolled

Registrational Study ★ NDA submission expected in 2020

Avapritinib: global first-in-class PDGFRA/KIT inhibitor

Targeted therapy with significant indication expansion potential

In Partnership With

blueprint MEDICINES

FDA U.S. FOOD & DRUG ADMINISTRATION

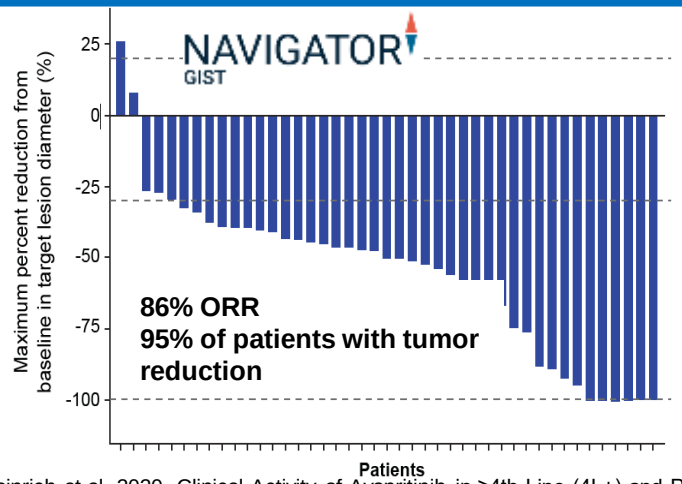
- ✓ Fast Track Review
- ✓ 2X Breakthrough Therapy Designation
- ✓ Priority Review

China Competition Overview

First China NDA Filing	2020
First in Class molecule	✓
# of Competitor(s) in Clinical Development	1
Lead over immediate competitor	>1 years
Global Patent Expiry	2034

Source: Globocan 2018; CStone analysis; Avapritinib CTOS 2018 Presentation

Encouraging Clinical Activity Shown in NAVIGATOR Registrational Phase 1 Study

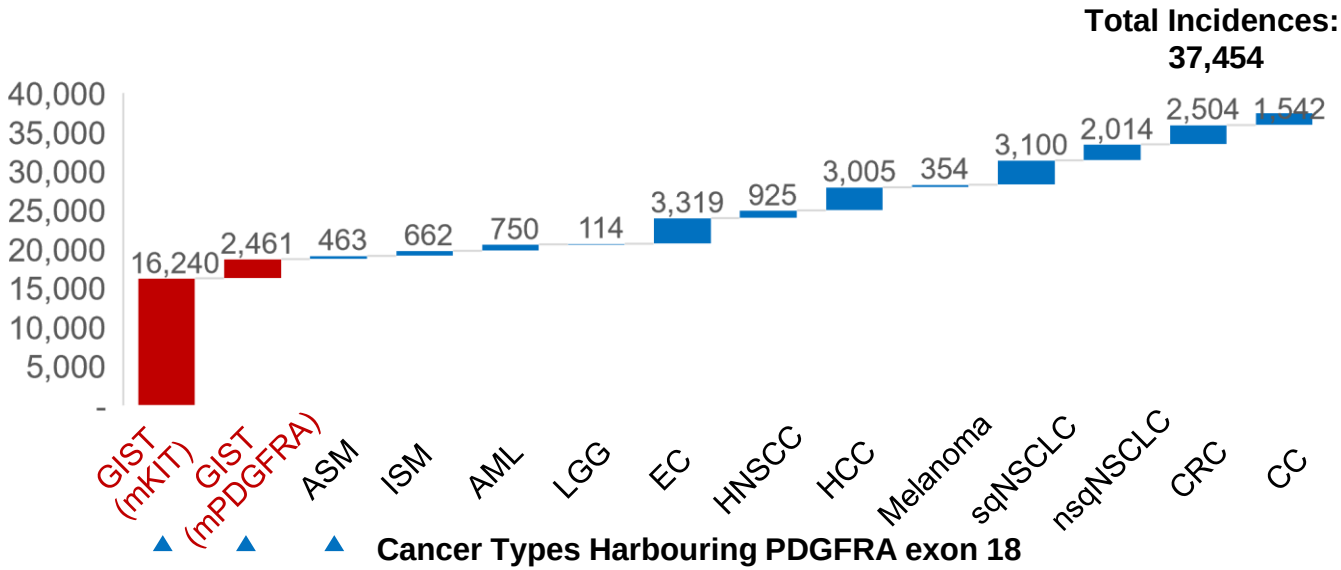


Safety Summary

- Most AEs were grade 1 or 2, with a higher incidence of commonly reported AEs in the 400 mg QD dose group compared with the 300 mg QD dose group.
- No treatment-related grade 5 AEs were reported.

Heinrich et al. 2020. Clinical Activity of Avapritinib in ≥4th Line (4L+) and PDGFRA Exon 18 Gastrointestinal Stromal Tumors (GIST). Poster presented at the Gastrointestinal Cancers Symposium, Jan 23 San Francisco, CA

Total Newly Diagnosed Addressable Incidences in China (2018)



▲ *Active Clinical Programs*

Cancer Types Harbouring PDGFRA exon 18 and/or KIT exons 9/11/13/14/17 mutations

Significant clinical progress – pralsetinib (CS3009)
Global and China development and regulatory status

Global

Partner	Drug Candidate	Target	Indications	Pivotal / Registrational Trial Patient Target Enrollment	U.S. NDA Submission	U.S. NDA Approval
blueprint MEDICINES	Pralsetinib	RET	NSCLC*	✓**	✓	
			2L MTC	✓	✓	

✓ Achieved ✓ To be expected in 2020

Greater China

Partner	Drug Candidate	Indications	Region	Mono /Combo	Study Initiation	Patient Enrollment	NDA Submission	NDA Approval
blueprint MEDICINES	Pralsetinib (CS3009)	NSCLC 2L	China	Mono	Joining global study		★	
		MTC 1L	China	Mono	Joining global study			
		NSCLC 1L	China	Mono	Joining global study			
		Basket cohort	China	Mono	Joining global study			

Registrational Study Registrational Potential Study ★ NDA submission expected in 2020

Note: * Blueprint Medicines initiated rolling NDA submission to the U.S. FDA and expect to complete rolling NDA submission in Q1 2020
** Evaluation in Phase 1 ARROW trial ongoing. Initiated site activation for Phase 3 AcceleRET Lung trial in Q1 2020

Pralsetinib: potentially first-in-class RET inhibitor

Targeted therapy with significant indication expansion potential

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In Partnership With

blueprint MEDICINES

FDA U.S. FOOD & DRUG ADMINISTRATION

✓ 2X Breakthrough Therapy Designation

China Competition Overview

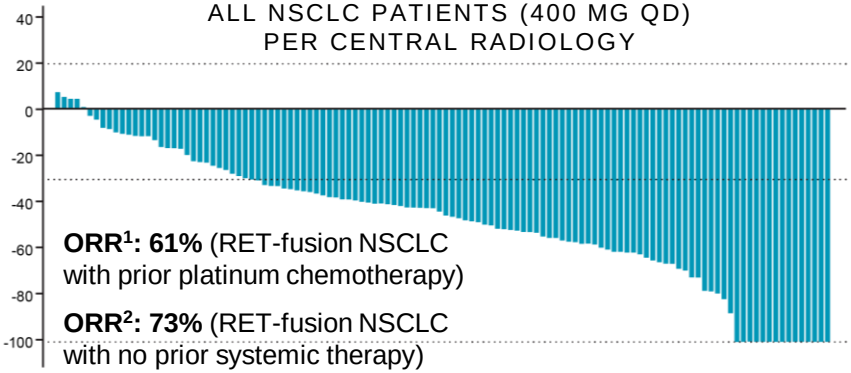
First China NDA Filing	2020
First in Class molecule	✓
# of Competitor(s) in Clinical Development	1
Lead over immediate competitor	~1 years
Global Patent Expiry	2036

*Eli Lilly's RETi currently not in China

Source: Globocan 2018; CStone analysis; Pralsetinib ASCO 2019 Presentation

Broad and Durable Antitumor Activity in Patients with RET Fusion+ NSCLC

ARROW



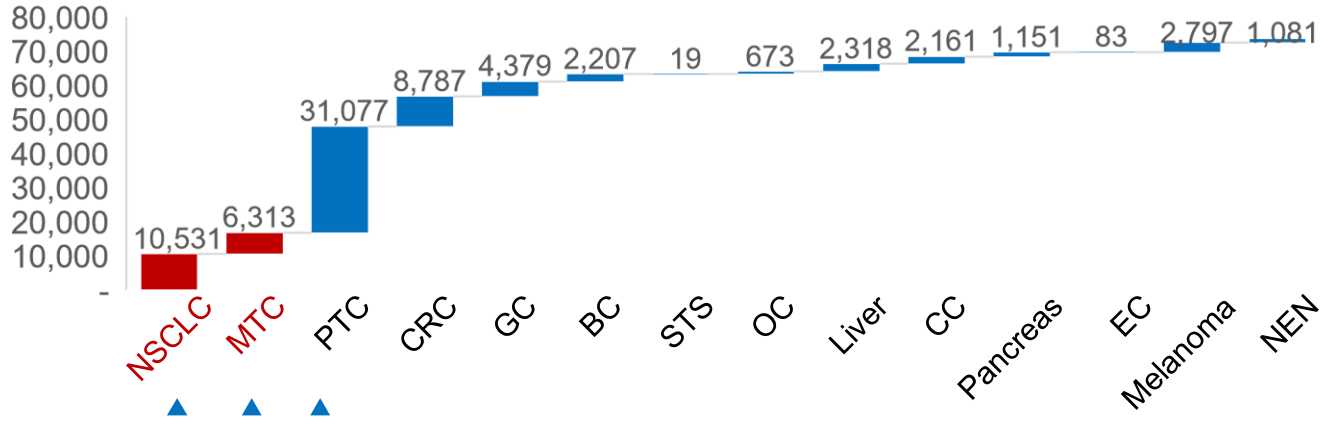
Safety Summary

- Safety results (N=354; 400 mg QD) were consistent with prior data; most reported AEs were grade 1 or 2"No treatment-related grade 5 AEs were reported.
- Overall, 4% of patients discontinued treatment due a treatment-related AE.

Top-line data from the Phase 1/2 ARROW trial in patients with RET fusion NSCLC. Data cutoff date: November 18, 2019.
1. Two responses pending confirmation. 2. All responses confirmed.

Total Newly Diagnosed Addressable Incidences in China (2018)

Total Incidences: 73,576



Cancer Types Harbouring RET Mutation

▲ *Active Clinical Programs*

Significant clinical progress on other clinical-stage assets

CS3005 (A2aR)	■ Conducting a Ph I trial for solid tumors as monotherapy in Australia/China and plan to initiate a combo with CS1003 (PD-1) thereafter
CS3002 (CDK4/6)	■ Conducting a Ph I trial for solid tumors as monotherapy in Australia/China and plan to initiate a combo with CS1003 (PD-1) thereafter
CS3003 (HDAC6)	■ IND/CTA approvals received in China and Australia in March 2019 and April 2019 respectively
CS3006 (MEK)	■ Conducting a Ph I trial in Australia and expect to complete the dose escalation portion in 1Q20 ■ Conducting a Ph I trial in China and expect to complete the dose escalation portion in 1Q20

Note: RP2D = recommended phase 2 dose

CStone is driving pipeline 2.0 for sustained growth

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Pipeline 1.0 → **GAP Analysis** → **Pipeline 2.0**

Balanced Portfolio

- De-risked I/O Backbone & Assets
- Precision Medicine
- Validated & Novel I/O Combination

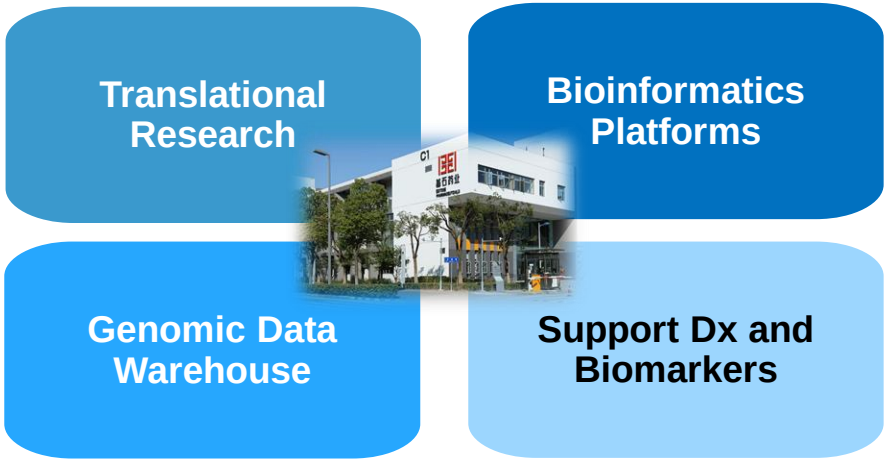
New Targets

- "Practice changing" potential?
- Convincing combo activity with PD-(L)1 or pipeline 1.0 candidates?
- "Disruptive" biology?

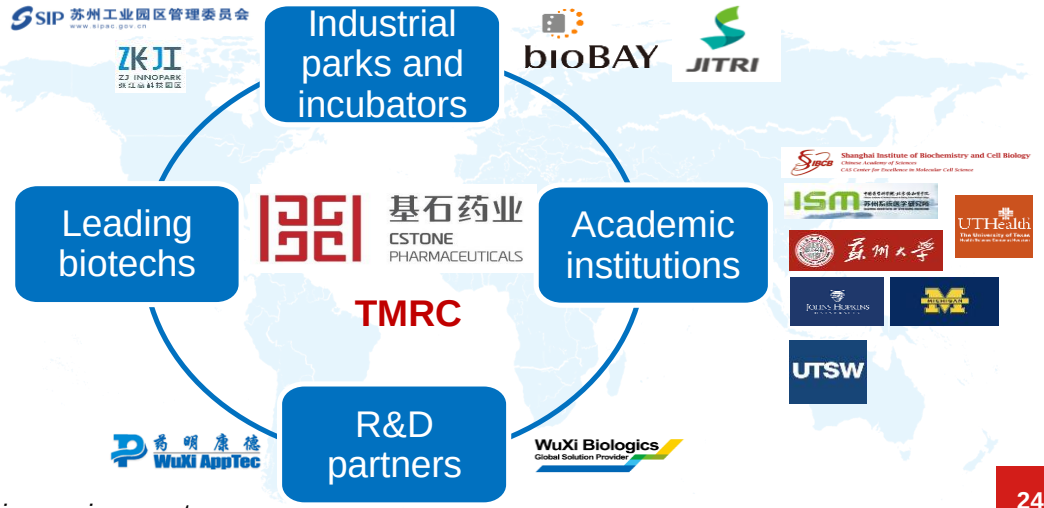
Advanced Portfolio

- FIC, Novel pathway
- FIC/FW/BIC multi-functional biologics/scaffolds
- Cancer vaccines
- TME

Fully integrated core functions at our Suzhou Translational Medical Research Center (TMRC)



Leverage the Ecosystem Centered around Our TMRC



Note: FIC = first in class; FW = first wave; BIC = best in class; TME = tumor microenvironment.

*Focus areas: n*ovel biology, multi-functional biologics, cancer vaccines

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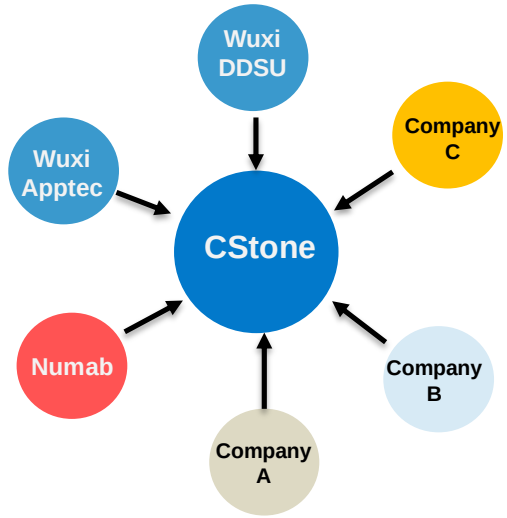
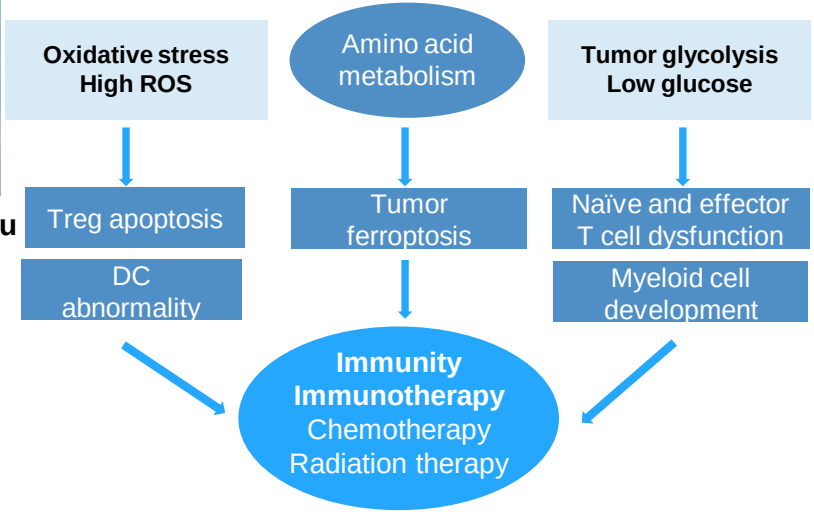
- **CStone Internal Discovery Platform**
  - "**Hub & Spoke**" models for internal projects
    - *CStone names the targets; Internal **PL&PM** to run projects*
    - *Lead discovery & optimization done at CRO or platform companies until **PCC**, then bring back to CStone*
  - Work with leading academic labs to identify lead candidates, then bring to CStone for development



Novel Biology

Weiping Zou MD, PhD



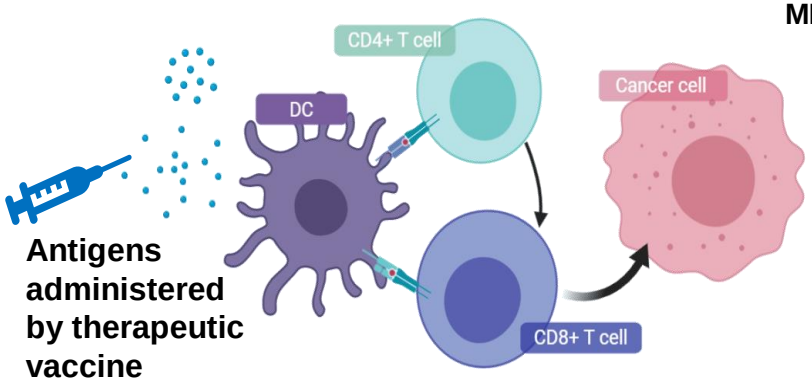
Targeting **ferroptosis pathway** may sensitize and improve immunotherapy efficacy

Cancer Vaccines

Internal Development

External Partnerships

Elizabeth Jaffee MD



Antigens administered by therapeutic vaccine

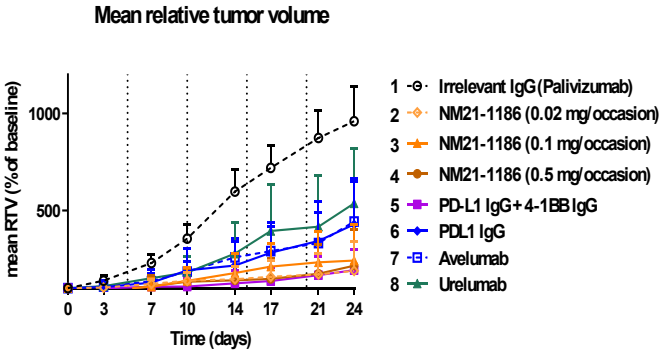
L Jiang et al. Ferroptosis as a p53-mediated activity during tumour suppression Nature 2015, 520: 57–62

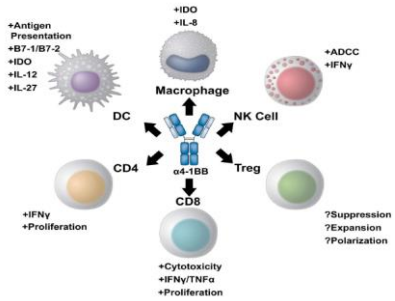
ND021 (PD-L1x4-1BBxHSA) has the potential to be the BIC molecule as the next generation PD-(L)1 inhibitor

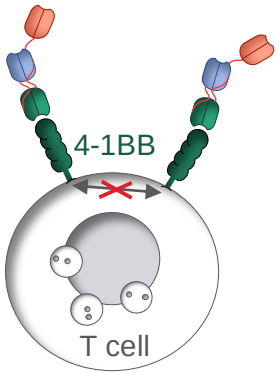
- Bridge new biology
- Improve therapeutic index & reduce unwanted toxic effects
- Expand combo options & improve administration convenience

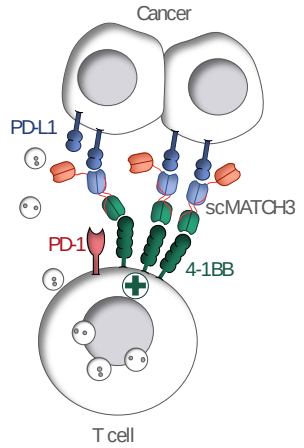
Six key features of ND021

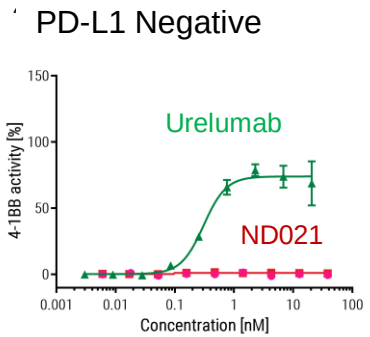
- Combination therapy in one molecule
  →expands combo options with SoC and other ICIs
- Monovalent 4-1BB binding remains inactive until PD-L1 engagement
  →maximizes safety
- Ultra-high affinity (pM) to PD-L1
  →broadens PD-L1+ tumor types
- Standard affinity to 4-1BB (nM)
  →ensures effective activation
- HSA extends T1/2 & 100% effector null
  →enables convenient dosing & eliminated undesirable FcgR-mediated activation
- MW ~70kDa, only half of conventional mAb
  →leads to better tumor penetration & efficacy

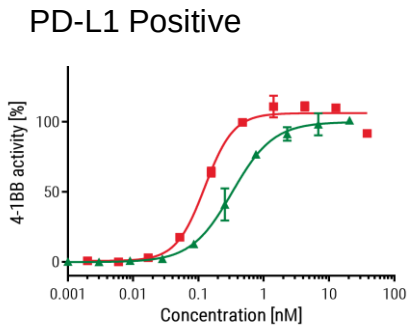












Strategically partnering with global and domestic leading biopharmaceutical companies in past two years

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1

5 FIC/BIC Assets from 3 Global Partners

agios

- Tibsovo (IDH1) 🏆

blueprint MEDICINES

- Avapritinib (KIT & PDGFRA) 🏆
- Pralsetinib (RET)
- Fisogatinib (FGFR4)

NUMAB Drug Innovators

- ND021

2

3 Clinical Collaborations Deals Focused on IO Combo Therapy

Zelgen

- CS1001 (PD-L1) + Donafenib

BAYER

- CS1001 (PD-L1) + Regorafenib

IMPACT Therapeutics

- CS1001 (PD-L1) + IMP4297

🏆 Received U.S. FDA approval after the in-licensing deal

Global collaboration deal with China focus to evaluate PD-L1 in combination with regorafenib in key indications

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BAYER

PD-L1 + Stivarga (regorafenib) tablets

Highlights

- First collaboration with a MNC pharma, one of the very few without PD-(L)1 – a vote of confidence in CStone and CS1001 (PD-L1)

Strategic Value

- Further strengthens our core strategy in IO combination therapy
- A big step forward for CStone's global strategy in case of positive data

Progress Update

- In December 2019, the first patient was dosed in a Phase Ib trial of CS1001 in combination with regorafenib in Australia

Regorafenib reported promising data with PD-1 in gastric cancer and colorectal cancer at ASCO 2019

Indication	Mono/Combo	ORR
Advanced GC	Pembro	13%[1]
	Rego + Nivo	**44%[2]**
pMMR/MSS CRC (95% of mCRC)	Pembro	0%[3]
	Rego	2%[4]
	Atezo	2%[4]
	Atezo + MEK	3%[4]
	Rego + Nivo	**33%[2]**

Note: 1. KEYNOTE059; 2. 2019 ASCO data, All respondents were Microsatellite stable (MSS); 3. 2015 ASCO data; 4. IMBlaze370
pMMR = mismatch repair proficient

We will focus on precision medicine in the near-term, followed by PD-(L)1 across China prevalent indications in the longer term

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Near-term (2020~2021)	Mid-term (2022~2025)	Long-term (2026~)
Expect 4 products Across 5 indications[1]	**Expect ~6 products Across 15 indications[1]**	**Expect ~13 potential approved products Across 20+ indications[1]**
AYVAKIT avapritinib tablets: PDGFRA GIST, 3L GIST	AYVAKIT avapritinib tablets: PDGFRA GIST, 3L GIST, 4L GIST	AYVAKIT avapritinib tablets: PDGFRA GIST, 3L GIST, 4L GIST
Pralsetinib: 2L NSCLC	Pralsetinib: 1L NSCLC 1L, 2L NSCLC, 1L MTC	Pralsetinib: 1L NSCLC, 2L NSCLC, 1L MTC
TIBSOVO ivosidenib: r/r AML	TIBSOVO ivosidenib: r/r AML, NIC AML	TIBSOVO ivosidenib: r/r AML, NIC AML
CS1001 (PD-L1): cHL OR NKTL	**CS1001 (PD-L1)**: cHL OR NKTL, S4 NSCLC, S3 NSCLC, ESCC, GC	**CS1001 (PD-L1)**: cHL OR NKTL, S4 NSCLC, S3 NSCLC, ESCC, GC
	PD-1: HCC	**PD-1**: HCC
	Fisogatinib: HCC	Fisogatinib: HCC
		CTLA-4; CDK4/6; A2A; PD-L1/4-1BB/HSA; CS1009, CS3004, CS2004

We are constantly filling our pipelines that fit with our pipeline 2.0 approach

Note:1. For in-licensed assets NDA approval time will depend on our partners' NDA approval time by US FDA

Shirley Zhao, a top commercial executive with 26 years experience to build and scale up commercial capabilities in Greater China

基石药业 CSTONE PHARMACEUTICALS

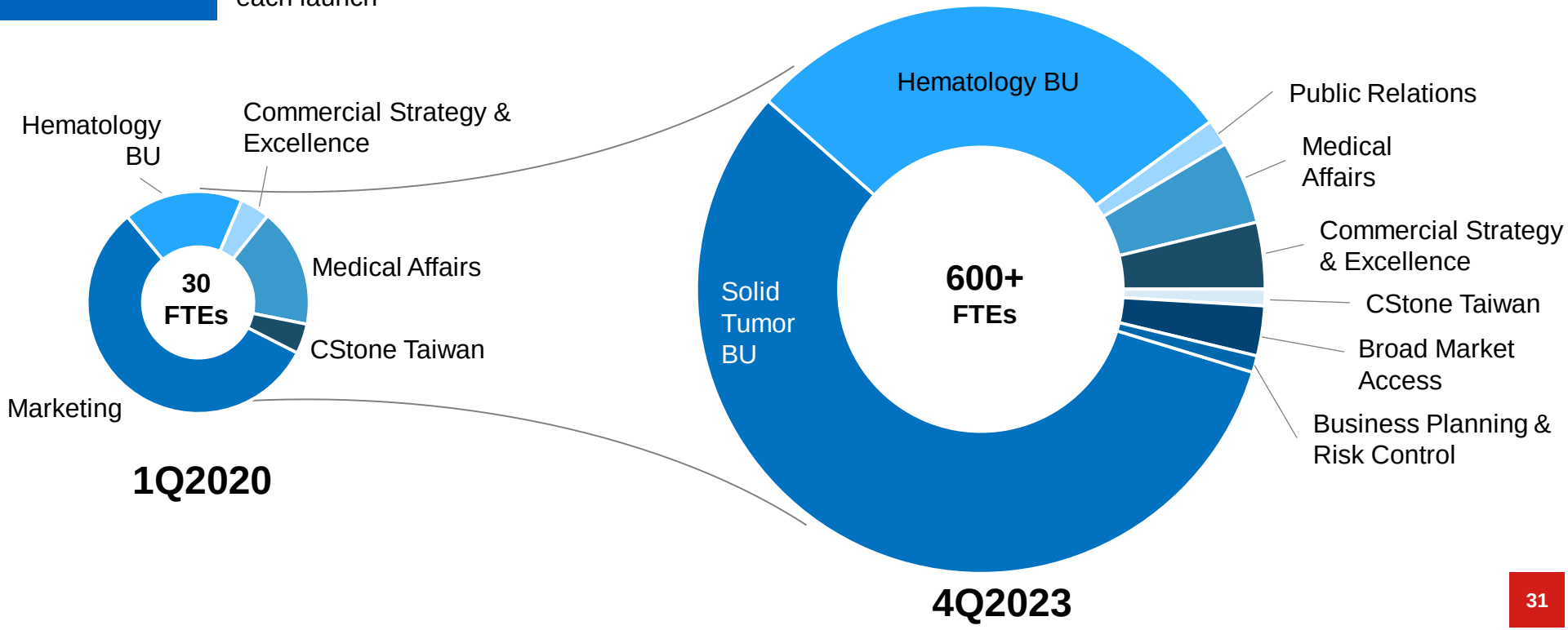
Greater China GM
& Head of Commercial

Shirley Zhao
MD, MBA

- Former **BMS** China mainland and HK General Manager (GM), brought China's first PD-1 (OPDIVO) to the market and led a $650M commercial operation
- Former **Allergan** China President, built China's commercial operation from $10M to $300M in 6 years, and become the 2nd biggest business in Allergan worldwide
- Former **Genzyme** China GM, successfully achieved registration milestones, and launched several rare disease drugs
- 10 years in **Eli Lilly** China as VP & Onco BU head, 5 years in BMS, launched Taxol, Paraplatine, Gemzar, Alimta
- Former **OB/GYN** physician, received **MD** training in Clinical Medicine from Tongji University, and obtained **MBA** from University of Leicester

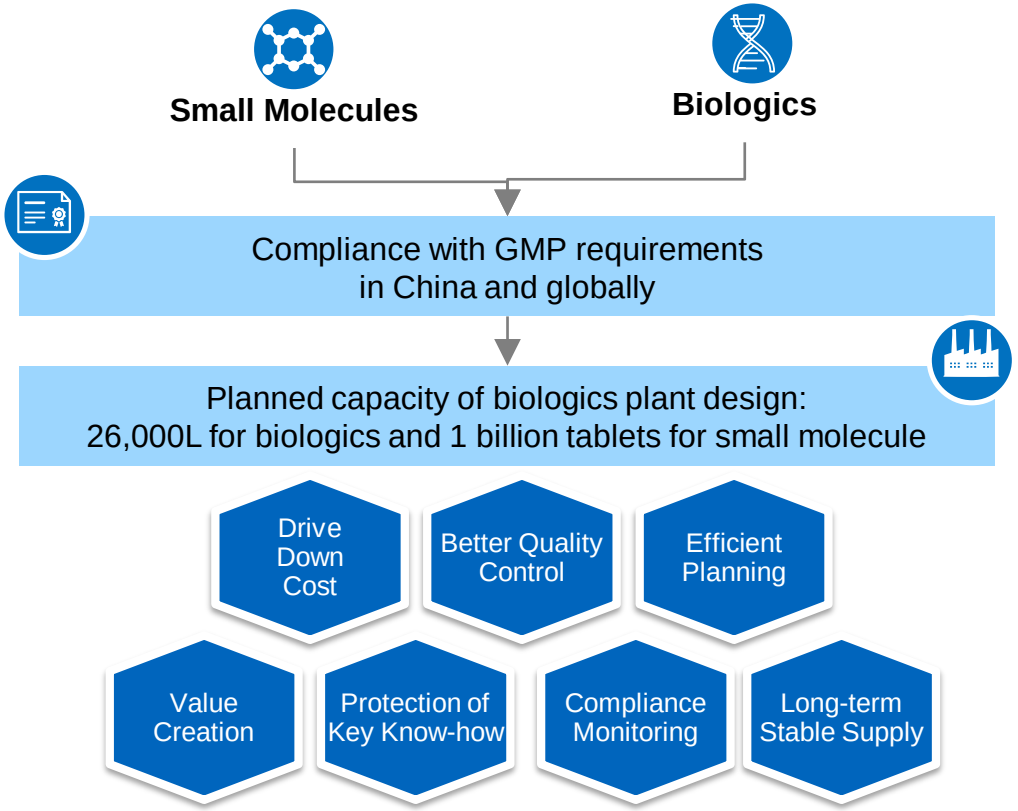
Full-fledged commercial organization being scaled up for multiple launches in the next 2 years

	Stage 1 2018-2021	Stage 2 2021-2023	Stage 3 2023+
Commercial model	1 ▪ **Establish commercial organization with core functions** for Greater China ▪ **Focus on self-build commercial model** while exploring potential value-driving strategic partnership	2 ▪ **Full launch of PD-L1 and in-licensed assets** across multiple major indications ▪ **Scale up Greater China** commercial presence	3 ▪ First wave of product **launches outside China[1],** preferably with **partner** ▪ **Scale up global** commercial organization to support pre-launch planning and launch
Launch & commercialization	**Build and scale up commercial capabilities** (Sales & Marketing; Access; Medical Affairs; Distribution; Commercial Excellence etc.) to support China and global launches, ramped up 18-24 months in advance of each launch		



Developing strong manufacturing capabilities for both biologics and small molecules

- In August, we entered into an agreement with Sungent (state-owned controlled by **Suzhou Industrial Park**) to build manufacturing facility
- Planned building area of approximately **100,000 sqm**
- Commissioned to a third party and is scheduled to **break ground in 2020**
- **Capability**: once completed, the complex will be equipped with integrated capabilities for R&D, Pilot Plant, and full commercial scale manufacturing
- **Planned capacity**: **26,000L** for macromolecule biologics and **1 billion tablets** and capsules for small molecule drugs
- **Strategic partnership with Wuxi Biologics** on clinical and commercial stage manufacturing



Financial summary in 2019

Cash position

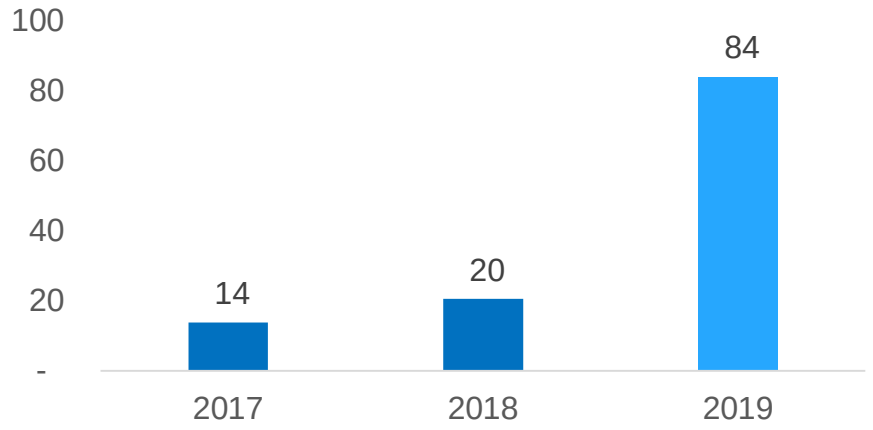
Cash Balance	■ **RMB2,726 million of cash, cash equivalents, and time deposits** as of December 31, 2019 vs. RMB1,463 million as of December 31, 2018 ■ Cash position increased by RMB1,263 million mainly due to proceeds from our IPO in February 2019
Sources of Cash	■ **IPO proceeds**: HK$2,394 million (post greenshoe) ■ **Other income**: RMB84 million, mainly consists of interests income from bank deposits and time deposits
Uses of Cash	■ **Adjusted R&D expenses[1] (non-IFRS)**: RMB1,189 million ■ **Adjusted administrative expenses[1] (non-IFRS)**: RMB138 million ■ **Listing expenses**: RMB18 million

1. Adjusted for share-based compensation

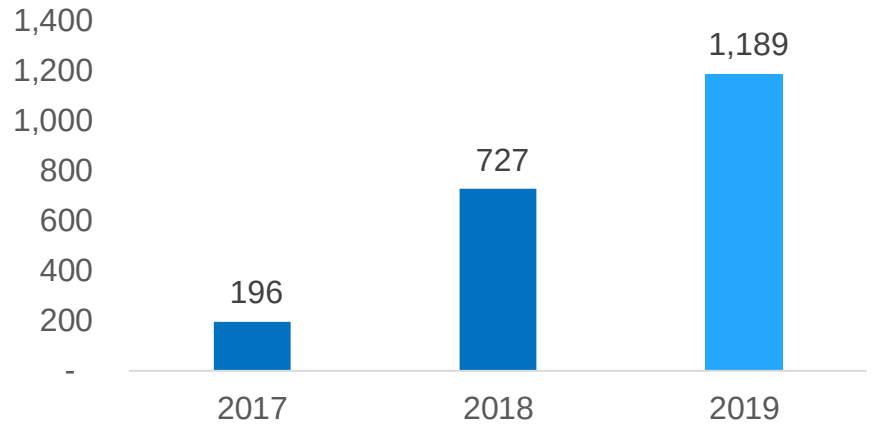
Financial summary in 2019 (continued)

Key income statement items

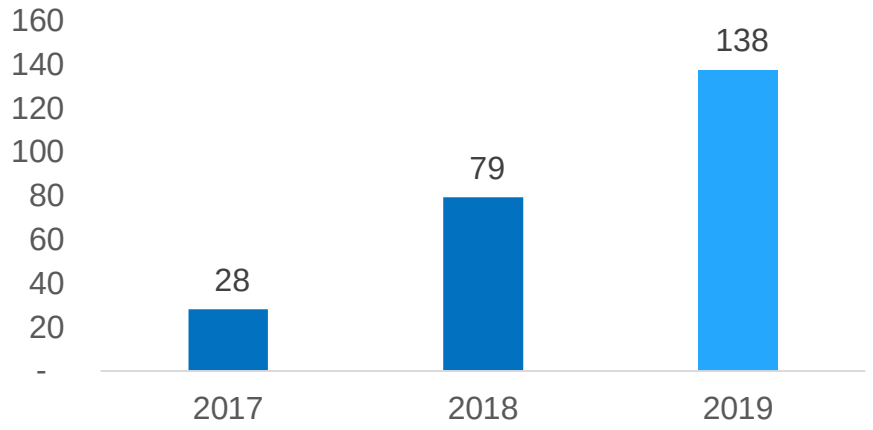
Other Income (RMB mm)



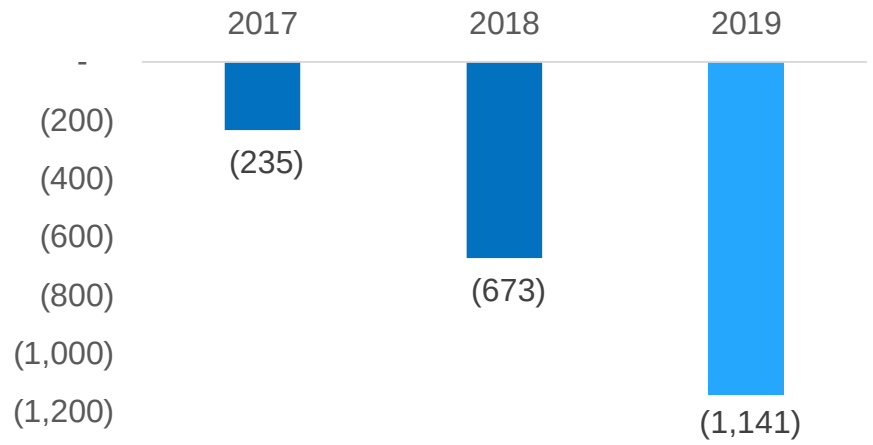
Adjusted R&D Expenses[1] (RMB mm)



Adjusted Administrative Expenses[1] (RMB mm)



Adjusted Loss for the Period[2] (RMB mm)



1. Adjusted for share-based compensation
2. Adjusted for share-based compensation and loss from fair value changes of the conversion feature of preferred shares

2019 performance in perspective

	2019 guidance	2019 actual performance
Clinical	**By the number** ■ **25+** clinical trials by year end ■ Including **10+** registrational studies ■ Approximately **10+** combination therapy trials **NDA timeline** ■ Taiwan NDA planned for **ivosidenib** in 2019 **Data release** ■ **CS1001 (PD-L1)** ■ **CS1003 (PD-1)** ■ **CS3006 (MEK)**	**By the number** ■ **28** clinical trials by year end ■ Including **13** registrational studies ■ **11** combination therapy trials **NDA timeline** ■ **Submitted ivosidenib** in Taiwan **Data release** ■ **CS1001 (PD-L1):** Ph 1a published at ASCO, Ph Ib and Ph II NKTL published at CSCO, ESMO, ASH ■ **CS1003 (PD-1):** Ph Ia data published at CSCO ■ **CS1002 (CTLA-4):** Ph Ia data published at CSCO
Others	■ **Research:** deliver **1-2 new molecule INDs** each year ■ **Manufacturing:** site selection in progress	■ **Research: CS3002 (CDK4/6)** and **CS3005 (A2aR)** entered clinical trials ■ **BD:** licensed ND021 from Numab and reached clinical collaboration with Bayer ■ **Manufacturing:** entered into an agreement to build manufacturing facility and global R&D headquarter in Suzhou ■ **Commercial**: China GM Shirley Zhao on board

Exciting year: 2020 will be a key inflection point for CStone, expecting the following major catalysts and goals

By year end 2020, **30+** clinical trials initiated, including **15** for registration

Pipeline	1 NDA approval	TIBSOVO Ivosidenib	■ r/r AML, Taiwan
	5 NDA submissions	PD-L1	■ cHL (potentially NKTL), China
		Avapritinib	■ PDGFRA exon 18 GIST, China ■ 3L GIST, China ■ PDGFRA exon 18 GIST, Taiwan
		Pralsetinib	■ 2L RET NSCLC, China
	7 Data readouts	PD-L1	■ **Stage IV NSCLC squamous and non-squamous** Ib trial data at ASCO 2020 ■ **Stage IV NSCLC** registrational trial data by Q3 2020 ■ **Stage III NSCLC** registrational trial data by Q4 2020 / Q1 2021
		Avapritinib	■ **3L GIST** registrational trial data by Q2 2020 ■ **GIST with PDGFRA exon 18** registrational trial data by Q2 2020
		Pralsetinib	■ **2L NSCLC** registrational trial data by Q3 2020 ■ **1L MTC** registrational trial data by Q4 2020
Commercial		Ivosidenib	■ Product launch in Taiwan for r/r AML
		Team	■ Build up commercial team to ~200 FTE
Business Development			■ Continue to explore value creation partnership opportunities with domestic and multinational players
Research			■ Our partner Numab to submit NM21-1480 (ND021) U.S. IND next week (March 2020)

Note: AML= Acute Myeloid Leukemia, cHL= Classical Hodgkin's Lymphoma, GIST = Gastrointestinal Stromal Tumor, NKTL = Natural Killer/T Cell Lymphoma, NSCLC = Non-small Cell Lung Cancer, MTC = Medullary Thyroid Cancer, r/r = Relapsed or Refractory

基石药业
CSTONE
PHARMACEUTICALS

Thank you!