Abbisko Therapeutics Announces an Exclusive Licensing-out Agreement for Pimicotinib (ABSK021)

Conference Call

DEC 2023

Agenda

- **Pimicotinib Transaction Summary**

Dr. Yao-Chang Xu

- **Pimicotinib Pipeline Summary**

Dr. Zhui Chen

- **Pimicotinib Transaction Details**

Dr. Zidong Zhang

- **Company Strategy & Other Pipeline**

Dr. Yao-Chang Xu

- **Q&A**

Pimicotinib(ABSK021) Transaction Summary

Transaction Logics

- **Maximize** the value and impact of Pimicotinib (ABSK021) **through partnership with global leading company for unmet medical needs of potential patients in China and globally**
- **Increase cash balance** and decrease expenditures to mitigate market uncertainty risks

Transaction Structure

- Granted Merck an exclusive license agreement to commercialize Pimicotinib in China(included HK, Macao and Taiwan) **with an option for rest of the world** and subsequent development rights upon the achievement of certain conditions
- Upon exercise of the commercialization option for the rest of the world, Merck also has the option to co-develop Pimicotinib in additional indications

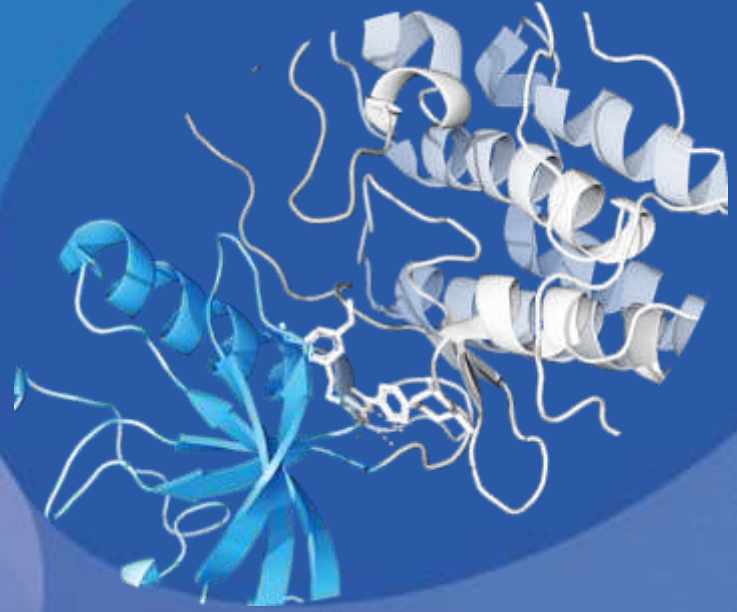
Transaction Financial Terms

- Pimicotinib(ABSK021) entered an exclusive agreement in China, included upfront payment, milestones and global option fee and double-digit% tiered royalties
  - **Upfront payment of $70m and total amount of $605.5m**
  - **When Merck exercises the global commercialization option, we will receive an additional option exercise fee**
  - We will receive additional payments for the achievement of certain development and commercial milestones and **double-digit tiered royalties on net sales** by Merck

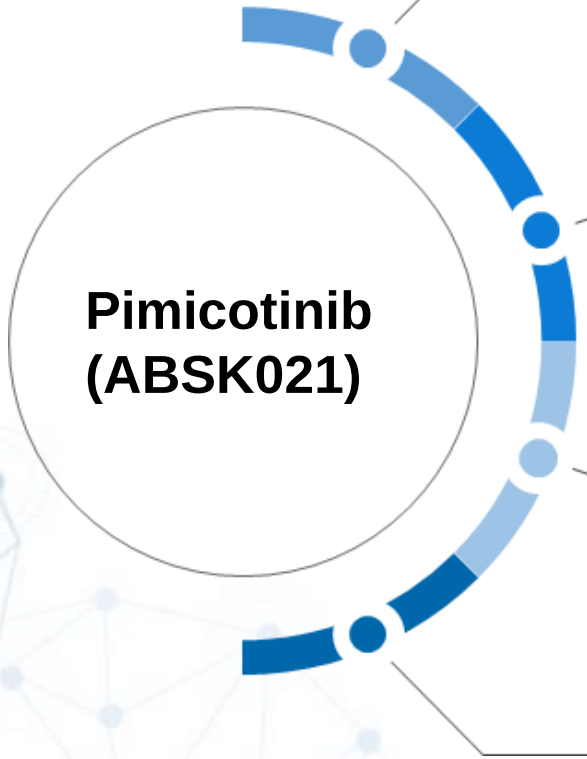
Post-transaction Strategy

- Strengthen **late stage R&D, clinical development, CMC, NDA submission and commercialization** to maximize the valued of Pimicotinib and **expand geographic coverage from China to global** through partnership
  - We continued to progress Pimicotinib to a global Phase III study in TGCT and other indications. Merck will advance commercialization in China

Pimicotinib（ABSK021）
CSF1-R’s Pipeline Summary



Summary of Pimicotinib (ABSK021)



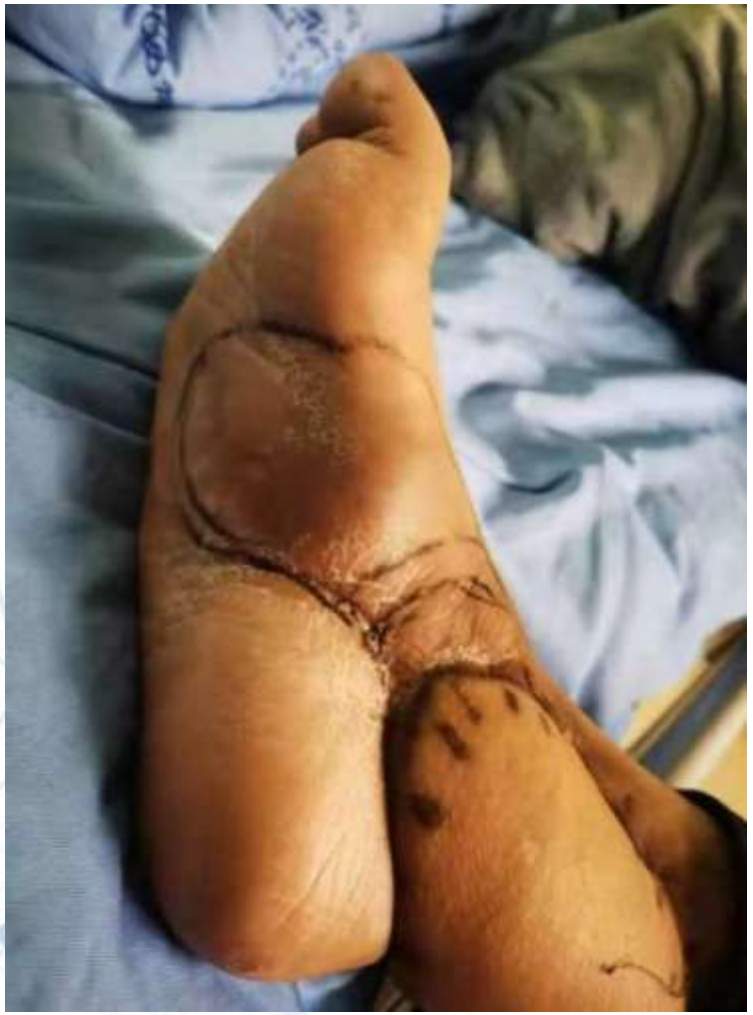
- **Breakthrough** Therapy Designation in **China & US**, and Priority Medicine (PRIME) Designation in **Europe**
- **Ph III (global MRCT)** for TGCT ongoing **in China**，**in North America and Europe**; NDA submission expected in 2024/25
- Potentially **BIC** in TGCT with **87% ORR** with 1-year follow and strong safety profile
- Expanding into other diseases: **PhII cGvHD** ongoing; **PhII pancreatic cancer** first patient dosed; **Ph1b for multiple solid tumors** ongoing

We Aim to Expand Pimicotinib (ABSK021) into Multiple CSF-1R-Dependent Therapeutic Areas with Multi-Billion Dollars Market Potential

Strategy	1 Accelerated development in TGCT	2 Expansion into other indications (many with potential orphan drug status)		3 Combo in broad oncology indications
Addressable patient no. (1)	TGCT ~60k ~14k	cGvHD	ALS ~43k ~19k	Solid Tumors >400k
Commercial potential (1)	>$1.5bn	>$3.5bn	>$1.5bn	>$6bn
Pimicotinib (ABSK021) Development Status	▪ 87% ORR, potential best-in-class ▪ BTD in US/CHN/Europe ▪ In Phase III US/CHN/Europe	▪ In Phase II	▪ Trials in planning	▪ 1L pancreatic study Phase II study ongoing

1. Based on market research and internal analysis

TGCT Is A Disease with Large Patient Population and Significant Unmet Medical Needs Globally



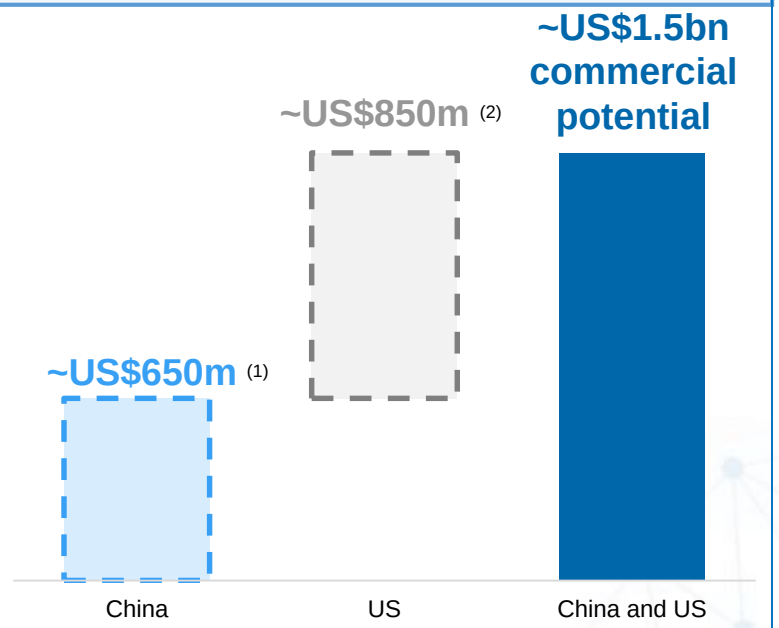
- Surgical resection is the standard treatment but with high recurrence rate.
- The only approved CSF-1R inhibitor, pexidartinib, has severe hepatotoxicity and was approved with black-box warning.

Commercial potential of CSF-1R inhibitors in TGCT (US$)

Annual incidence [3]:

~14,000 in U.S.

~60,000 in China



1. Based on internal analysis
2. Based on estimates from Deciphera Pharmaceuticals
3. Based on annual incidence rate of 43 cases per million people

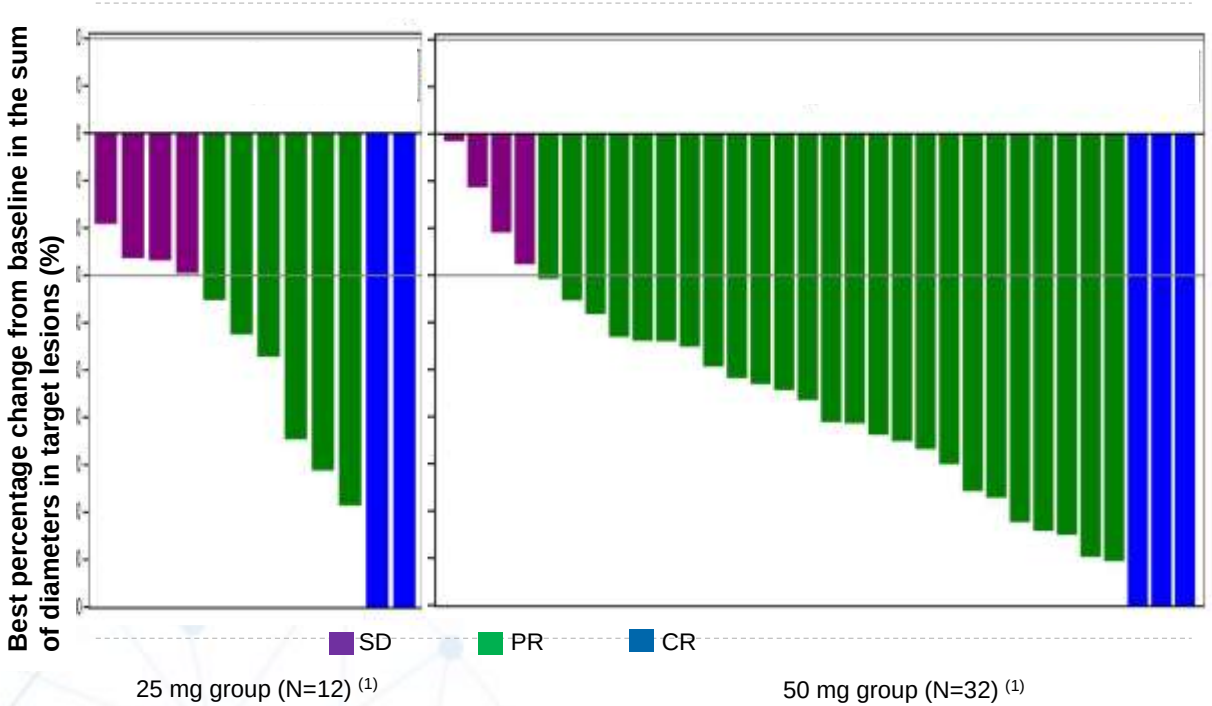
Pimicotinib (ABSK021) showed improved ORR at 87.5% and a clean safety profile during one-year follow up

Efficacy

- The preliminary ORR: **87.5%** (28/32, QD 50 mg) (by IRC, including **3 CR**), 100% DCR
- Dose dependency: ORR **66.7%** (by IRC, including **2 CR**), compared with **87.5%**

Safety

- Favorable safety profile at both cohorts with **no apparent hepatotoxicity**
- **83.9%** of patients remained on treatment, and median treatment duration were **13.6** months in 50 mg QD; the longest treatment duration was 17.5 months



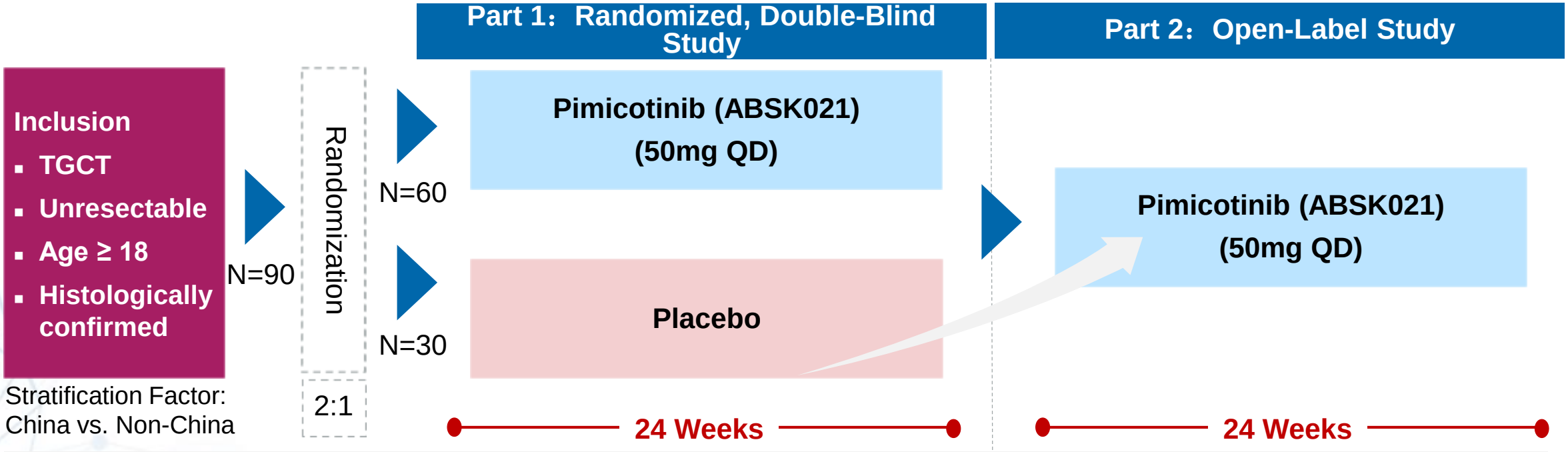
TEAE Rate ≥15% Preferred Term(2), n (%)	Grade 3/4	
	25 mg QD (N=12)	50 mg QD (N=44)
LDH increased	0	0
Blood CPK increased	0	1 (2.3)*
α-HBDH increased	0	0
AST increased	0	0
Amylase increased	0	0
ALT increased	0	0
Rash	0	0
Pruritis	0	0
Face edema	0	0
Dizziness	0	0
Somnolence	0	0

1. All 12 TGCT patients in 25 mg cohort have completed at least one post-dose tumor response assessment by IRC. And 32 out of 44 TGCT patients in 50mg cohort have completed at least one post-dose tumor response assessment by IRC. Cut-off date: May2023.

2. Cut-off date: May2023. * This patient experienced extensive work-out.

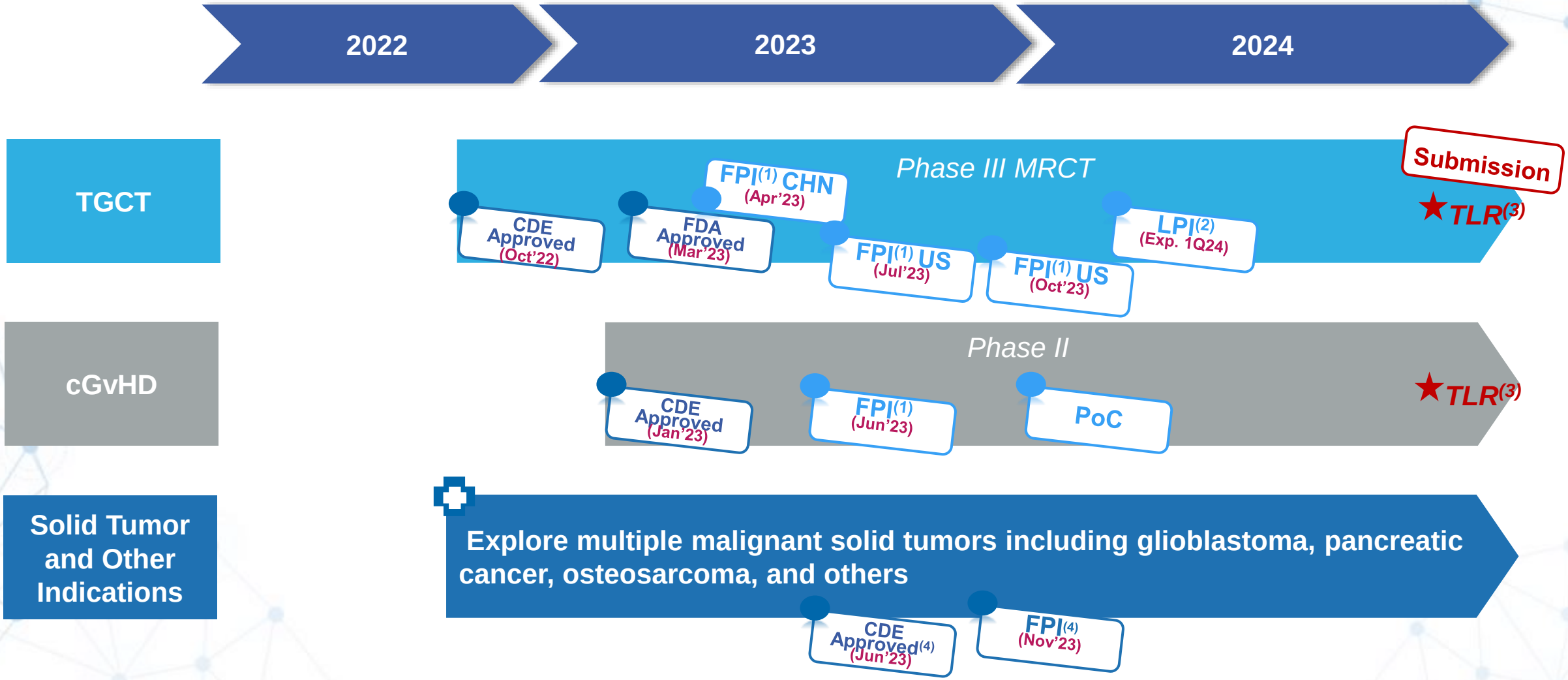
The Global Multicenter Phase III Trial for TGCT Is Approved by the FDA ,CDE&EMA and Patient Enrollment Has Started in US, China and Europe

- A randomized, double-blind, placebo-controlled Phase III clinical study to evaluate the efficacy and safety of Pimicotinib (ABSK021) at the dose of 50mg QD in patients with Tenosynovial Giant Cell Tumor



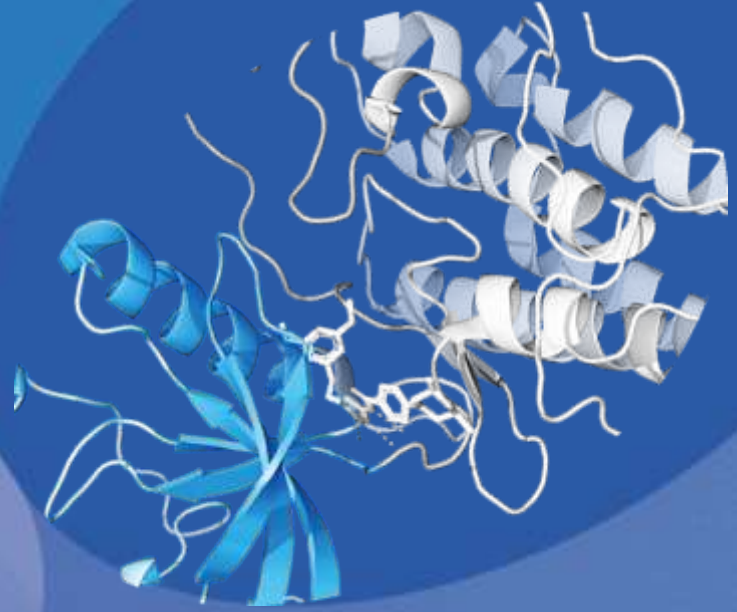
- **Primary Endpoint:**
  - 25-Week ORR by Blinded Independent Review Committee (BIRC) based on RECIST 1.1
- **Secondary Endpoints:**
  - 25-Week ORR by BIRC based on Tumor Volume Score (TVS)
  - Mean change from baseline in Range of Motion (ROM) of the affected joint at Week 25

Pimicotinib (ABSK021) Clinical Progress and Future Development Plan



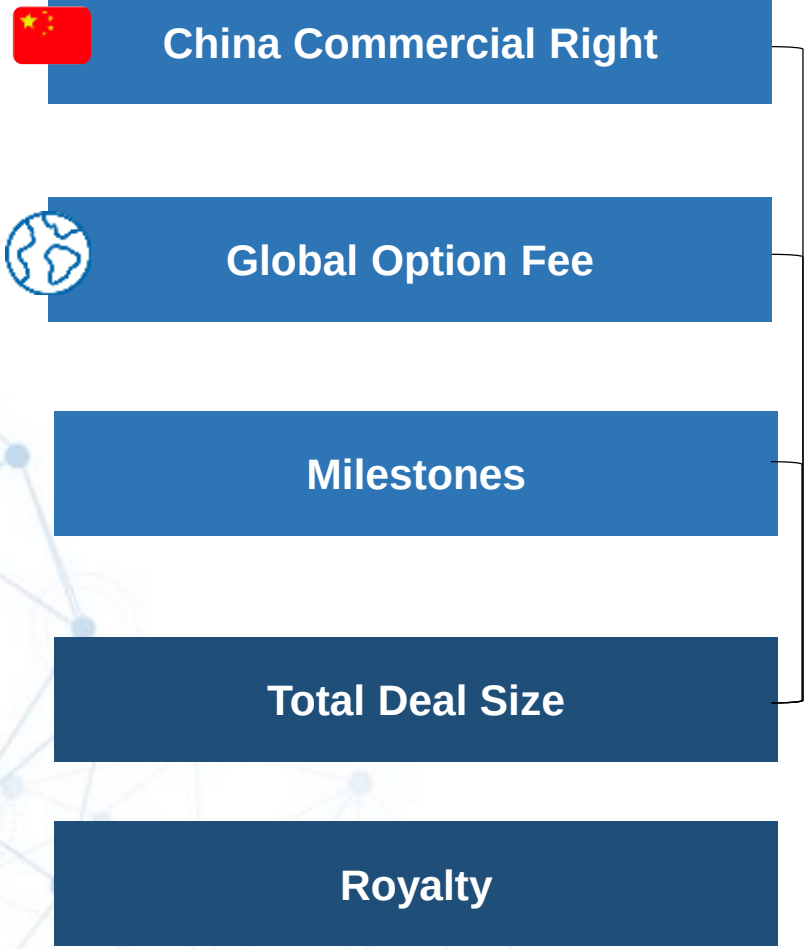
1. FPI: first patient in. 2. LPI: last patient in. 3. TLR: topline result. 4. Pancreatic Cancer.

Pimicotinib（ABSK021）Transaction Details



Abbisko Therapeutics Co., Ltd.

Pimicotinib's Financial Terms Summary



$70 million
Mainland China, Hong Kong, Macau and Taiwan

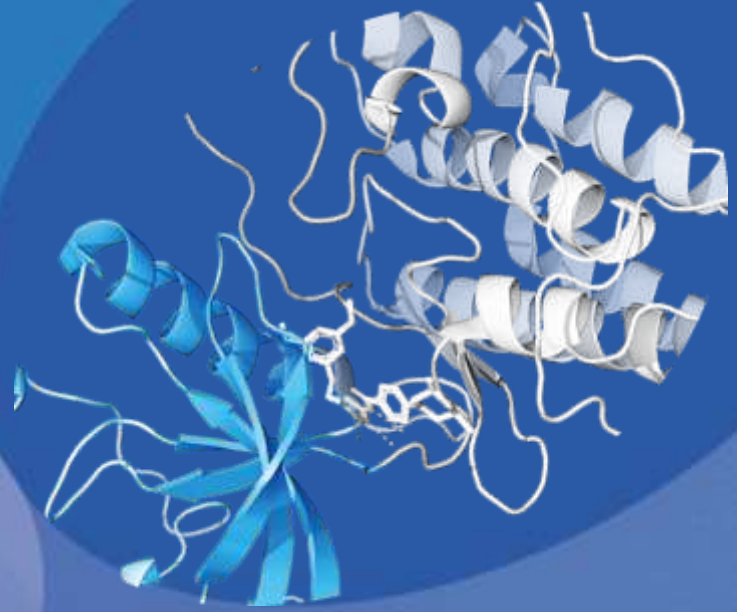
Additional Option Exercise Fee
When Merck exercises the Global Commercialization Option

Development & Commercialization Milestones
Available, details not disclosed

Up to $605.5 million
Aggregated upfront, exercising payment, and development & commercialization milestones

Double-digit percentage (%)

Company Strategy & Core Pipeline



Abbisko: Transitioning from Late Clinical Stage to Commercial Stage

Early Research Stage

- In-house discovered **16 PCCs with best-in-class potential**, **delivering 2~4 PCCs per year**:
  - R&D focus，R&D personnel **>150**;
  - Founded by top-notch industry veterans from Hansoh, Novartis etc., with discovery track record of blockbusters such as Almonertinib

Mid & Late Clinical Stage

- Entered into mid & late clinical stage:
  - **Pimicotinib (ABSK021) TGCT NDA submission expected in 2024**;
  - **At least 2 proof-of-concept assets** into critical clinical stage (**Irpagratinib (ABSK011)、Fexagratinib (ABSK091) from FGFR franchise; oral PD-L1**);
- Built a clinical team of ～**100** personnel; and a CMC laboratory fully operational with GLP certification;
- License-out of assets with cash inflow (**1-2 deals per year**)

Commercial Stage

- Commercialization: enter **overseas markets through partnership**, develop domestic market through collaborations/self-built team;
- **Focus on cancer**, with potential expansion into other major disease areas;
- Become a **fully-fledged commercial stage biopharmaceutical** driven by product sales and BD out-licensing

2016-2022 | **2022-2025** | **2025 and beyond**

Our Oncology-Focused Pipeline Consists of BIC & FIC Assets, CSF-1R Exclusively Licensed-out to Merck, FGFR Franchise in POC, and Early-Stage Assets for BD and Clinics

CSF-1R (ABSK021) Near Commercialization

1. First TGCT indication in Phase III:
- **ORR: 87% with 1-year follow**, BTD in US/CHN/Europe, potential BIC;
- Phase III in MRCT, for NMPA & FDA approval;
- Expected completion in early 2024, commercialization in 25/26, large patient population and potential

2. Expansion into other disease areas:
- cGvHD Phase II in progress, data expected by 2024;

3. Active exploration in solid tumor areas:
- Trials planned in pancreatic cancer, osteosarcoma, glioblastoma

4. License-out agreement with Merck
- Upfront **$70M** and total amount of **$605.5m**
- Possibly receive an additional option exercise fee
- Receive additional payments for the achievement of certain development and commercial milestones and double-digit tiered royalties on net sales by Merck

FGFR Franchise In POC

1. Huge unmet medical need of FGFR:
- ~1.9 million cases per year of FGFR aberrations globally, mainly in lung cancer, breast cancer, urothelial cancer, bile duct cancer, etc.;
- FGFR4 mutation in ~30% HCC patients（>350,000 worldwide）

2. Strong efficacy data:
- Irpagratinib (ABSK011) demonstrated strong efficacy in 2L HCC patients with high expression of FGF19, **ORR~40.7%**

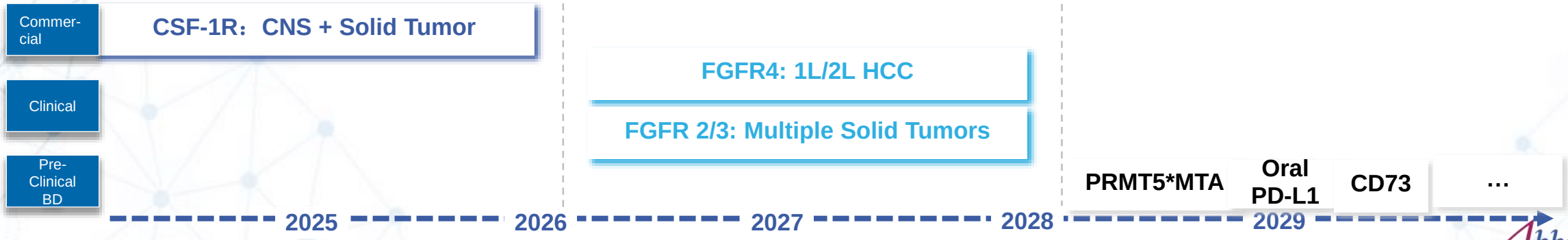
3. High effectiveness against FGFR3:
- Fexagratinib (ABSK091) presented ORR of 31%-44% in 2L UC in FGFR3 mutants

Early-Stage Assets For BD and Clinics

1. Collaboration with global pharmaceutical:
- Partner with Eli Lilly in early stage development in non-oncology chronic disease areas

2. Advancing early-stage targets into clinics:
- Explore BD deals for early-stage assets, such as KRAS, oral PD-L1, 4th-gen EGFR, 2nd-gen Exon20, CD73, FGFR

3. BD deals for pre-clinical assets to generate early cash inflow:
- 2~4 PCCs per year, along with continuous BD deals to generate early cash flow, to expand indications and regions



Our 10 Clinical Pipeline and 6 Preclinical Pipeline

Programs	Targets	Indications	Mono/Combo Therapy	IND	Phase I/Ia	Phase Ib/II	Phase III/NDA	Commercial Rights	Partner
Pimicotinib (ABSK021)	CSF-1R	TGCT ★	Mono					Global	
		cGvHD	Mono					Global	MERCK
		Solid Tumors	Mono/Combo					Global	
		ALS	Mono		Partner			Ex-Mainland China, HK and Macau	
Irpagratinib (ABSK011)	FGFR4	FGF19+HCC	Mono					Global	
			Combo			Combo with Roche anti-PD-L1 atezolizumab			
Fexagratinib (ABSK091)	pan-FGFR	FGFRalt UC	Mono				Partner	Global	AstraZeneca
			Combo			Combo with BeiGene anti-PD-1 tislelizumab			
		Other Solid Tumors	Mono					Global	
ABSK061	FGFR2/3	Solid Tumors	Mono					Global	
ABSK121	pan-FGFR mut.	Solid Tumors	Mono					Global	
ABSK112	EGFR Exon20	NSCLC	Mono					Global	
ABSK043	PD-L1 (oral)	Multiple Tumors	Mono					Global	
ABSK081	CXCR4	TNBC	Combo			Combo with Junshi anti-PD-1 toripalimab		Greater China	X4
		WHIM	Mono				Partner		

★ Breakthrough Therapy Designation (BTD/PRIME) ;

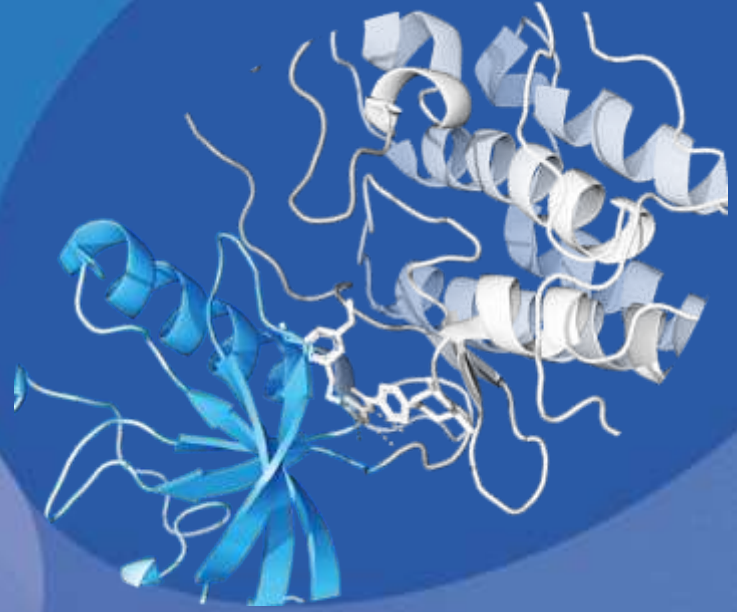
Abbreviations: ALS = amyotrophic lateral sclerosis; cGvHD = chronic graft-versus-host disease ; FGFRalt = FGFR altered; HCC = hepatocellular carcinoma; NSCLC = non-small cell lung cancer; RMS = rhabdomyosarcoma; TGCT = tenosynovial giant cell tumor; TNBC = triple-negative breast cancer; UC = urothelial cancer; WHIM = warts, hypogammaglobulinemia, infections and myelokathexis

Our Pipeline (Preclinical)

Programs	Targets	Indications	Mono/Combo Therapy	Lead Optimization/PCC	IND-Enabling	IND	Commercial Rights	Partner
ABSK051	CD73	Multiple Tumors	Combo				Global	
ABSK012	FGFR4 mut.	RMS and Other Solid Tumors	Mono				Global	
ABK3376	EGFR-C797S	EGFRm NSCLC	Mono/Combo	Mono/Combo with Allist Furmonertinib mesylate		*Partner*	Ex-China	艾力斯
ABSK071	KRAS	Solid Tumors	Mono				Global	
P131	MTA Cooperative PRMT5	Multiple Tumors	Mono				Global	
P141	Undisclosed	Multiple Tumors	Mono				Global	
P151	Undisclosed	Non-oncology	Mono/Combo				Global	Lilly

Abbreviations: ALS = amyotrophic lateral scierosis; cGvHD = chronic graft-versus-host disease ; FGFRalt = FGFR altered; HCC = hepatocellular carcinoma; NSCLC = non-small cell lung cancer; RMS = rhabdomyosarcoma; TGCT = tenosynovial giant cell tumor; TNBC = triple-negative breast cancer; UC = urothelial cancer; WHIM = warts, hypogammaglobulinemia, infections and myelokathexis

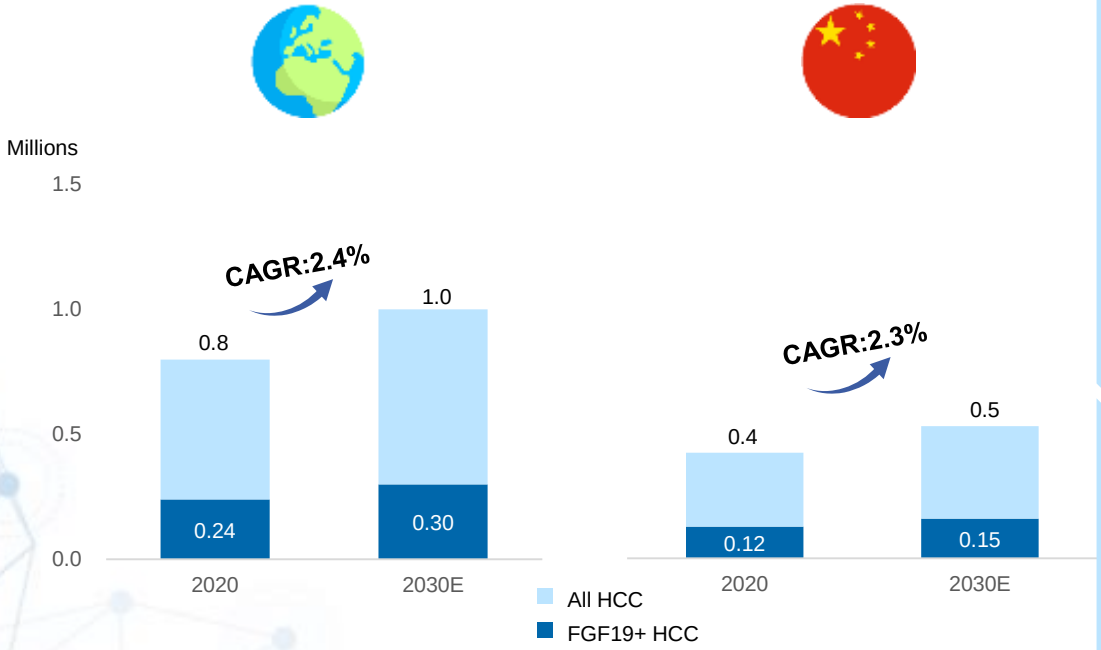
Other Core Assets



HCC: High Unmet Medical Needs from High Incidence Rate and Limited Treatment Options

HCC: 1m new patients Globally and 50% in China, FGF19+ HCC: ~30% of all HCC

New HCC Cases



Current Therapy Featuring IO & VEGF, With Limited Efficacy

	IO	VEGF
1L	Atezolizuma + Bevacizumab mOS 19.2m, mPFS 6.9m, ORR 29.8%	
1L	Tremelimumab + Durvalumab mOS 16.4m, mPFS 3.8m, ORR 20.1%	
1L		Lenvatinib mOS 13.6m, mPFS 7.3m, ORR 19%
1L		Sorafenib mOS 10.7m, mPFS 5.5m, ORR 2%
2L+	Nivolumab + Ipilimumab* mOS 22.8m, ORR 32%	
2L+	Pembrolizumab* mOS 13.2m, mPFS 4.9m, ORR 18%	
2L+		Regorafenib mOS 10.6m, mPFS 3.4m, ORR 7%
2L+		Cabozantinib mOS 10.2m, mPFS 5.2m, ORR 4%
2L+		Ramucirumab mOS 8.5m, mPFS 2.8m, ORR 4.6%

* Accelerated approval

Irpagratinib(ABSK011) Promising Efficacy Profile in Phase Ib Trial

Tumor Response in Prior Treated FGF19+ HCC Pts by Investigator Assessment (RECIST V1.1)

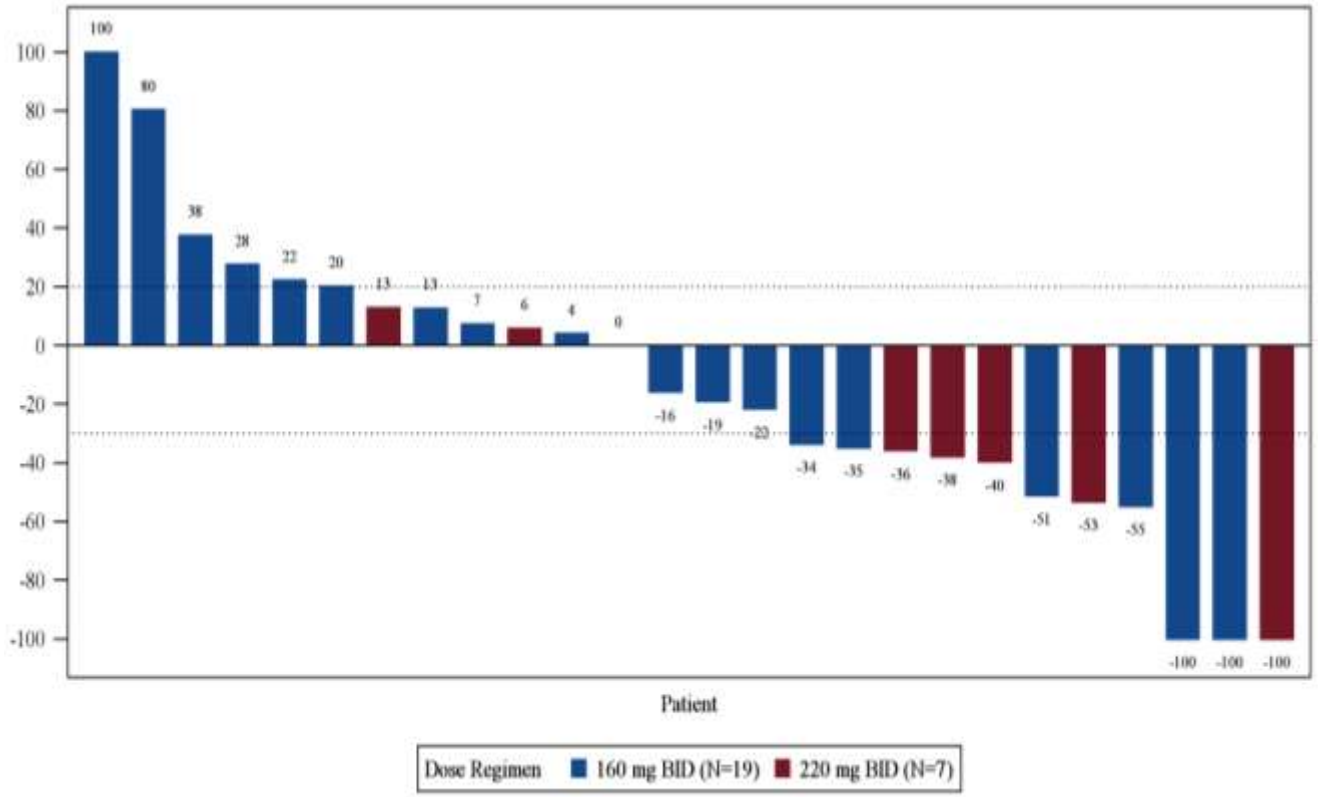
Response	QD	BID		
	180 mg QD （N=15)	160 mg BID (N=20)	220 mg BID (N=7)	Total (N=27)
BOR, n (%)				
CR	0	1(5)	0	1(3.7)
PR*	2 (13.3)	5 (25)	5 (71.4)	10 (37.0)
SD	10 (66.7)	6 (30.0)	2 (28.6)	8 (29.6)
Overall response rate*, n (%)	2 (13.3)	**6 (30.0)**	**5 (71.4)**	**11 (40.7)**
Disease control rate, n (%)	12 (80.0)	12 (60.0)	7 (100.0)	19 (70.4)

* including unconfirmed PR

The preliminary efficacy in FGF19+ HCC pts with prior therapies in BID cohorts:

- **The ORR was 40.7%.** In 26 evaluated patients, 14 observed with tumor shrinkage, including 3 complete response
- Median follow-up was 3.7 m, and mPFS was 3.9 m
  - mPFS in 220 mg BID was not yet mature
- The longest duration of response (DoR) was 9.6 m and mDoR was not yet mature, with 5 of 11 responses ongoing

Best Percentage Change in Sum of Diameters of Target Lesions in Prior Treated FGF19+ HCC Pts of BID Cohorts

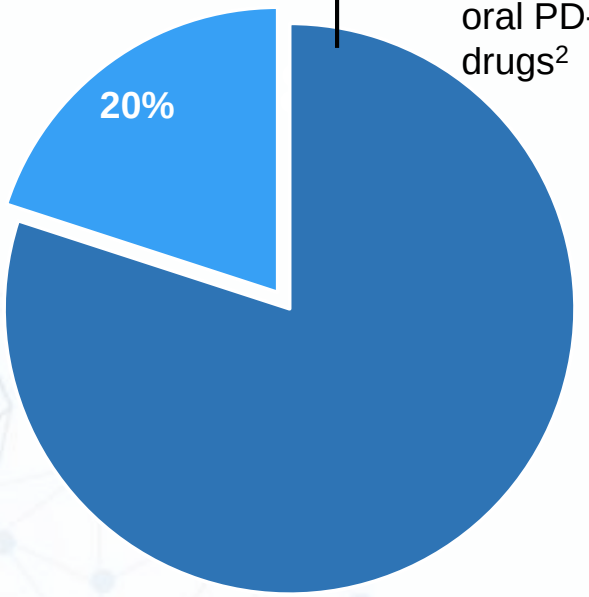


- Two pts obtained an overall response of PR, of whom the target lesions were assessed as CR, the non-target lesions were non-CR/non-PD, and no new lesions were observed.

Oral PD-L1 Small-molecule Has a Blockbuster Market Potential

80% of PD-(L)1 Market Can be Amenable to Oral Small Molecule Inhibitor

~$58Bn[1]



Significant Advantage of Oral PD-L1 Small Molecule Inhibitor

1 Oral formulation/ adjustable dosing schemes

- Ease of use, no intravascular (IV) costs
- Oral-oral combo

2 Improved tissue penetration

- Potential of better efficacy

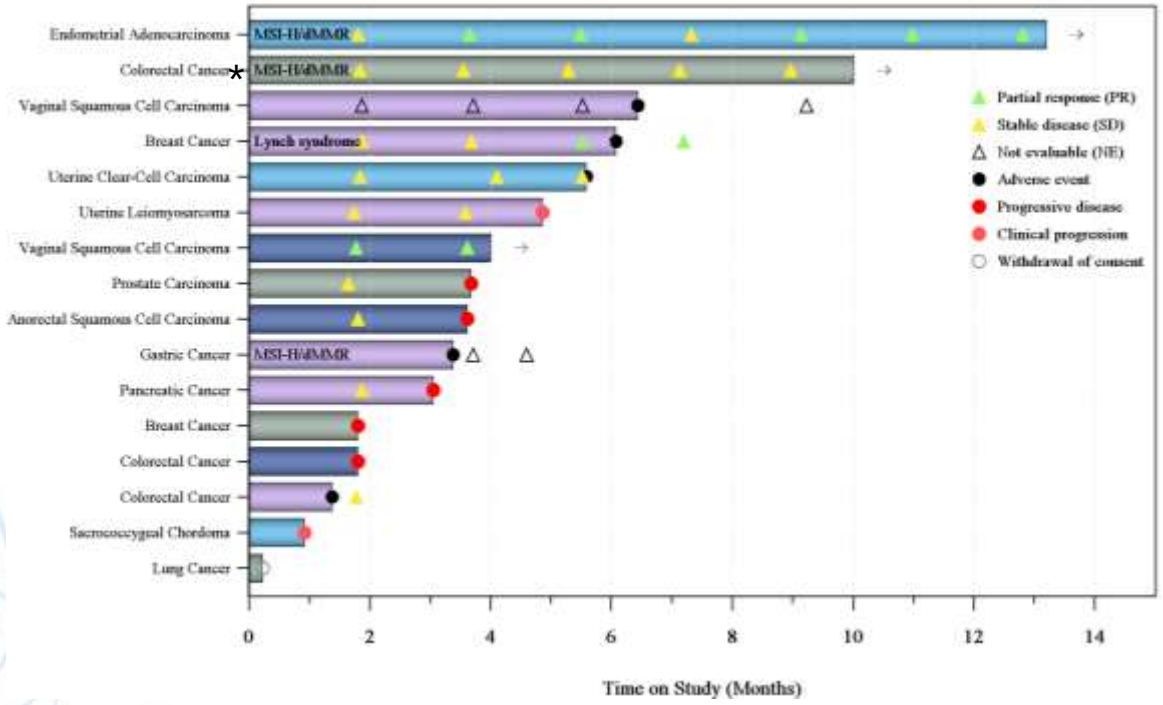
3 Non-immunogenicity

- Rapid titration
- irAE management

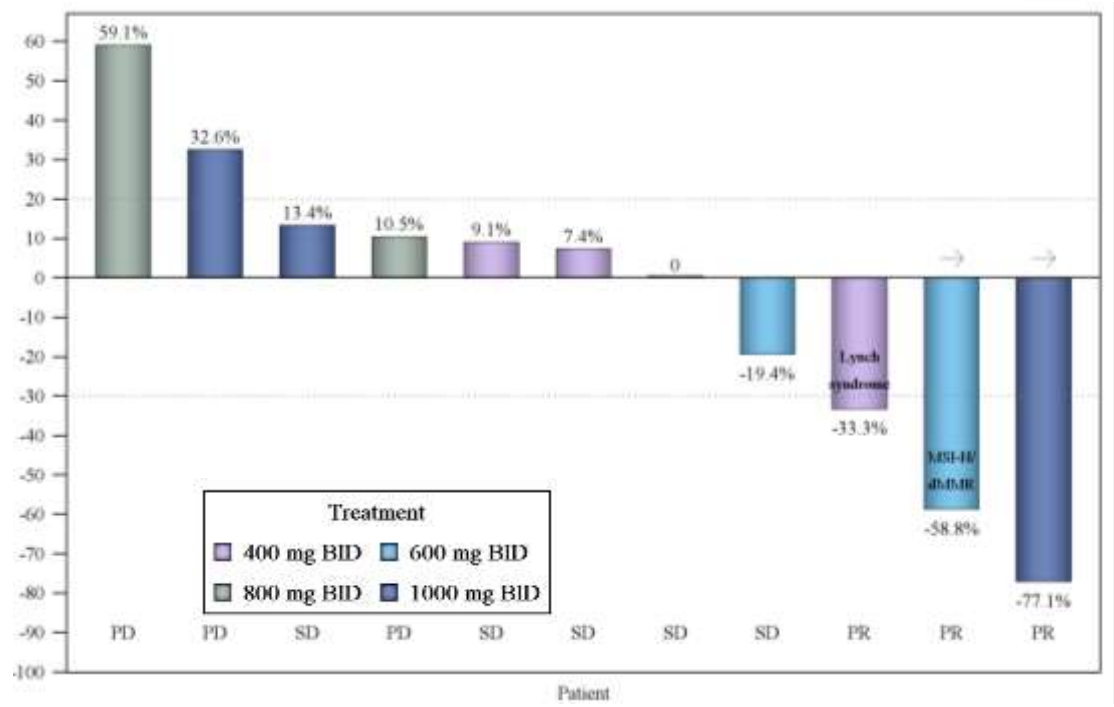
1. IQVIA White Paper-In the Eye of the Storm: PD-(L)1 Inhibitors Weathering Turbulence, 2022; 2. Based on Incyte company presentation, and with the assumption that oral PD-1 will be most likely to capture PD-(L)1 market in the mono therapy and combination setting with another oral agent, but not in combination with other injectables as this regimen still requires in-office visit

ABSK043 Promising Efficacy Profile in Preliminary Clinical Trial

Time on Treatment and Response



Best Percentage Change in Sum of Diameters of Target Lesions



Among 16 patients from BID dosing cohorts, **ORR ~27%** (11 tumor responses could be evaluated and 3 IO-naïve patients reached objective response)

- One endometrial carcinoma patient with MSI-H/dMMR (600mg BID) achieved confirmed partial response (PR) and has been on treatment for over 1 year
- Another breast cancer patient with Lynch syndrome (400mg BID) confirmed PR although discontinued due to Gr2 rash
- The third patient with vaginal squamous cell carcinoma treated 1000mg BID obtained confirmed PR (-77%) and is still on treatment

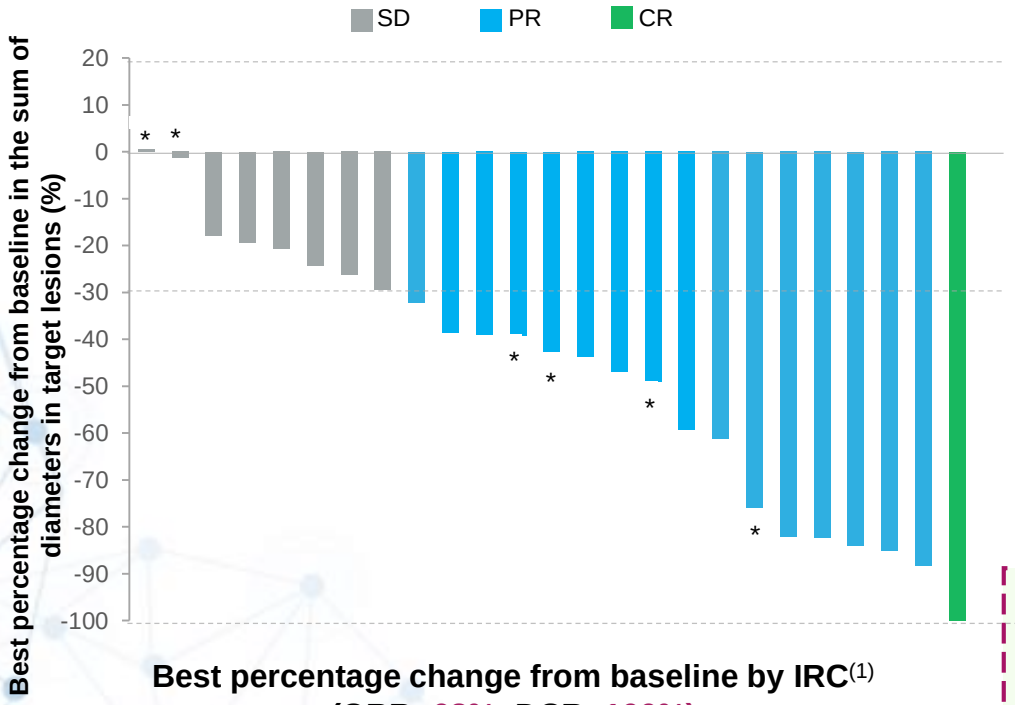
Pimicotinib(ABSK021) BD done, as we continue to advance our planned milestones

Pipeline	Target	Clinical Trial	Stage	Event	2023 Target	2023 Action
Clinical candidates						
Pimicotinib (ABSK021)	CSF-1R	TGCT	Phase III	✓ US Pivotal Trial Design Approval	1H	Mar'23
				✓ Global MRCT Pivotal Trial to Start	1H	Apr'23 CHN FPI Jul'23 US FPI
				✓ Extended Phase Ib Efficacy/Safety Results	1H	May'23 ASCO
		cGvHD	Phase II	▪ Preliminary Data Readout	2H	Jun'23 FPI
Irpagratinib (ABSK011)	FGFR4	2L HCC, mono	Phase Ib	✓ Extended Efficacy/Safety Results Including 2nd Dose Expansion	2H	Oct'23 ESMO
		1L/2L HCC, combo	Phase II	▪ Preliminary Data Readout	2H	2H
Fexagratinib (ABSK091)	Pan-FGFR	2L UC, mono	Phase II	▪ Extended Efficacy/Safety Results	2H	2H
ABSK043	PD-L1	Solid tumors	Phase I	✓ Preliminary Efficacy/Safety Results Readout	2H	Oct'23 ESMO
ABSK061	FGFR2/3	Solid tumors	Phase I	▪ Preliminary Phase Ia Data	2H	2H
ABSK121	FGFR mut.	Solid tumors	Phase I	✓ IND Approval in China ✓ FPI	1H 2H	Feb'23 2H
IND-enabling candidates						
ABSK051	CD73	Multiple tumors	IND-enabling	✓ IND Filing	2H	Nov'23
ABSK012	FGFR4 mut.	RMS and/or HCC	IND-enabling	▪ IND Filing	1H	2H
ABSK112	EGFR Exon20	NSCLC	Phase I	✓ IND Approval from FDA in US	2H	Jul'23


Thanks
Abbisko

Appedix1:Pimicotinib (ABSK021) Has Demonstrated Potentially Best-in-Class Efficacy

- The preliminary ORR: **68.0%** (17/25, 95%CI: 46.50%-85.05%) (by IRC based on RECIST1.1)
- **1 CR** and **16 PR** within 6 months in patients receiving 50mg QD treatment (out of 25 patients)
- **100%** preliminary disease control rate (“DCR”)



Best percentage change from baseline by IRC(1)
(ORR: 68%; DCR: 100%)

	Pimicotinib Abbisko		Pexidartinib(2) Daiichi Sankyo		Vimseltinib(2) Deciphera
Trial	Phase Ib (NCT04192344)	Trial	ENLIVEN study-Part 1 (NCT02371369)	Trial	Phase II, Cohort A (NCT03069469)
Patient no.	25	Patient no.	61	Patient no.	46
Dosage regimen	50mg QD	Dosage regimen	400mg BID	Dosage regimen	30mg BIW
ORR at 25 week (%)	**68%**	ORR at 25 week (%)	39%	ORR at 25 week (%)	38%

ASCO’23 Updated**77.4%**

1. * Represents tumor response at week 13. 25 out of 32 TGCT patients have completed at least one post-dose tumor response assessment by IRC. Cut-off date: 21 Sep 2022.
2. Data of pexidartinib comes from Tap WD et al (Lancet. 2019;394(10197):478-487); Data of Vimseltinib comes from Blay JY et al (1509P, ESMO. 2022; 33: S1236-1237).

Appedix2:Pmicotinib (ABSK021) Has Also Demonstrated Potentially Best-in-Class Safety Profile

	Pimicotinib Abbisko
Trial	Phase Ib (NCT04192344)
Patient no.	32
Dosage regimen	50mg QD

	Pexidartinib[1] Daiichi Sankyo
Trial	ENLIVEN study-Part 1 (NCT02371369)
Patient no.	61
Dosage regimen	400mg BID

	Vimseltinib[1] Deciphera
Trial	Phase II, Cohort A (NCT03069469)
Patient no.	46
Dosage regimen	30mg BIW

Any G3/4 TEAEs [n (%)]

CPK increased	1 (3%)
Pyrexia	1 (3%)
Rash	1 (3%)

Any G3/4 TEAEs [n (%)]

AST increased	6 (10%)
ALT increased	6 (10%)
ALP increased	4 (7%)
Hypertension	3 (5%)
Arthralgia	2 (3%)
Vomiting	1 (2%)
Rash	1 (2%)
Dizziness	1 (2%)
Periorbital edema	1 (2%)
Lactate dehydrogenase increase	1 (2%)

Any G3/4 TEAEs [n (%)]

CPK increased	20 (44%)
Asthenia	1 (2%)
Rash maculopapular	1 (2%)

1. Data of Pexidartinib comes from Tap WD et al (Lancet. 2019;394(10197):478-487); Data of Vimseltinib comes from Blay JY et al (1509P, ESMO. 2022; 33: S1236-1237).