

CANbridge
Pharmaceuticals



FY2022 Annual Results Presentation

March 2023

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Our Vision



To be a **Global
Biopharmaceutical
Company**

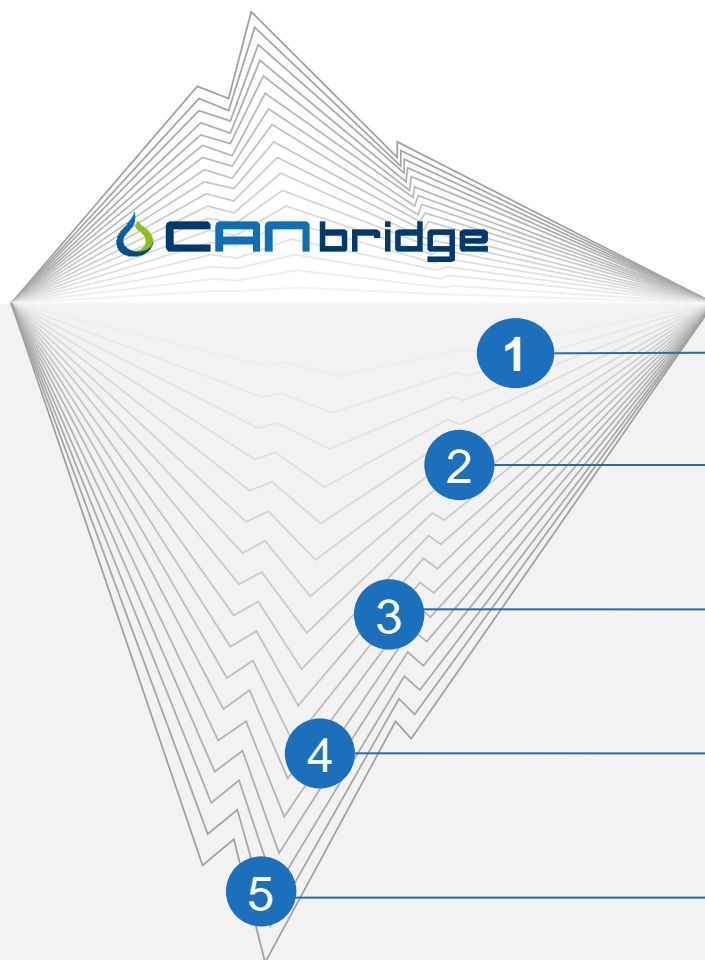


Delivering **Life-
changing Therapies
to Patients**



Built Upon
**a Foundation
in China**

Key Investment Highlights



Positioned to drive rapid and comprehensive product development and market access in China and globally

A comprehensive portfolio of rare disease-focused therapies with significant revenue potential

Track record of sourcing and developing innovative and validated therapies

A rare disease pioneer dedicated to addressing vast and unmet medical needs

Visionary management team with deep experience in developing and commercializing rare disease therapies globally

Experienced Management Team

Strong global management team with deep industry experience and a track record of commercializing rare disease treatments



Dr. James Qun Xue

Founder
Chairman of the Board
Executive Director
Chief Executive Officer

- **Veteran entrepreneur** with **22+ years** of experience in medical and pharmaceutical companies
- Former Founding General Manager of Genzyme China
- **Deputy Director General at CHARD, Vice Chair of R&D Committee of China Pharmaceutical Innovation and Research Development Association**



Dr. Gerald Cox

Chief Development Strategist
Interim Chief Medical Officer

- **21 years** of biotechnology executive management experience
- Former CMO at Editas Medicine and VP at Genzyme
- Made major contributions to 4 INDs and 3 orphan drug marketing authorizations for serious and life-threatening diseases that have **generated US\$ 3.0+ billion revenue for Genzyme**



Glenn Hassan

Chief Financial Officer

- **15+ years** of extensive banking, investment, and strategy consulting experience in the healthcare sector globally
- Former Director of Healthcare Investment Banking at China Renaissance
- **Veteran public market healthcare investor** at leading firms, including Citadel and Fidelity Management



Marcelo Cheresky

Chief Commercial Officer

- **~20 years** of business leadership experience in the biotechnology industry with **in-depth industry knowledge and extensive execution capabilities**
- Previous employment at leading biopharmaceutical companies such as Bioverativ, Ultragenyx, Synageva Biopharma and Genzyme



Yijun Lu

General Manager of CANbridge China

- Seasoned business executive with extensive experience and outstanding performance in **oncology and rare disease areas**
- Former Head of Hemophilia and Rare Disease at Takeda China, with a track record of leading the launch and development of rare disease products



Experienced Management Team



Chris Chen

Vice President of
Human Resources



Pauline Li

Senior Vice
President of Clinical
Development and
Operations



Bettie Li

Senior Director &
Head of Finance
Operation and
Controller



Qian Ma

Head of Legal and
Compliance,
Joint Company
Secretaries and
Board Secretary



Stella Mao

Senior Director,
Public Affairs



Shirley Yue

Senior Director,
Procurement and
Supply Chain



Rebecca Zhang

Senior Vice
President of
Regulatory Affairs



Wei Zhang

Senior Director &
China Head of CMC
Department

Part

01

Business Overview



A Rapidly Evolving Market for Developing Rare Diseases Products

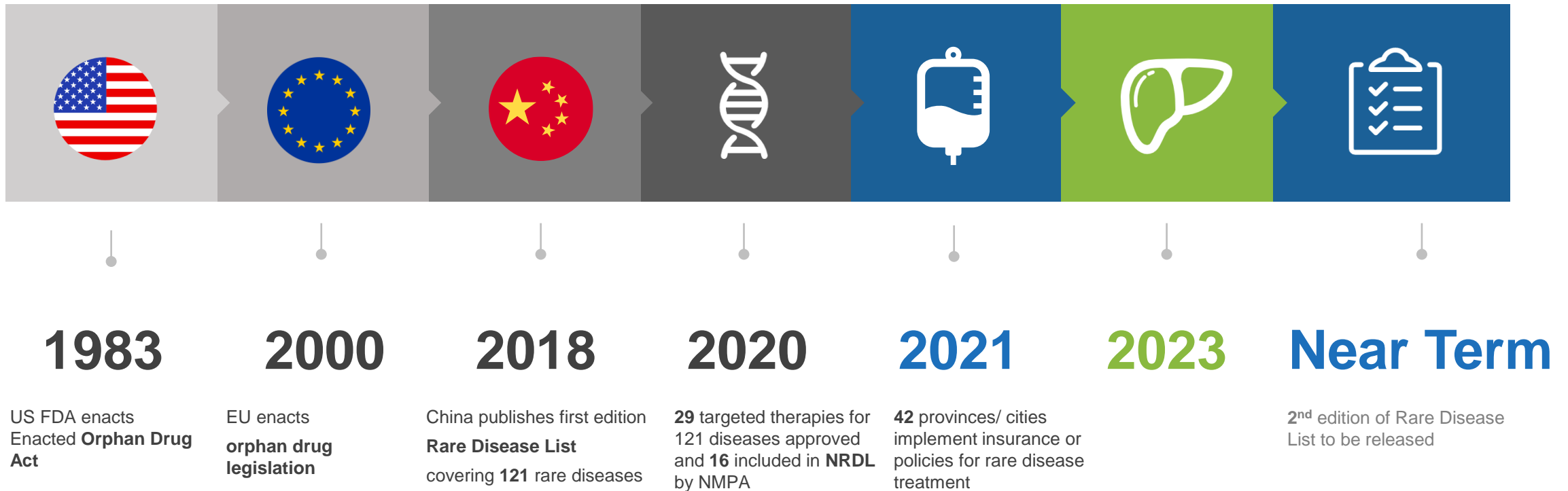
Emerging Favorable Regulatory Framework

 **CANbridge**
Potential Commercial Path 

Hunterase launch
in self-pay market

Potential Livmarli
launch in China

Special rare disease funding
Global Expansion to Further
Improve ASP



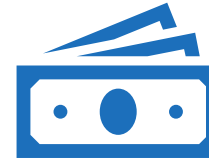
A Rapidly Evolving Market for Developing Rare Diseases Products

Rare Disease Drug Development Incentives



Faster approval timelines

- Drugs **approvable** under certain conditions **with overseas clinical data**, regardless of approval status in other markets
- Rare disease drugs have **smaller** and **less expensive clinical trials**
- **Expedited regulatory process** with agreed upon trial designs for a **speedy approval**
- China CDE issued “**Technical Guidelines for Clinical Development of Rare Disease Drugs**” in 2022



Financial incentives

- New China plan to slash the **Value Added Tax (VAT) by 80%** on select disease therapies and active pharmaceutical ingredients
- NMPA proposes granting new RD drug market exclusivity up to **7 years**































Increased awareness






- National Network of Rare Diseases (NNRD) and the National Rare Diseases Case Reporting System of China (NRDCRS) were formed in 2019 to better **identify and treat patients** with rare diseases, which **aids clinical trial enrollment and commercialization** in China

Our Comprehensive and Diversified Pipeline

A portfolio of biologics, small molecules and gene therapy solutions with validated mechanisms of action, targeting some of the most prevalent rare diseases and rare oncology indications with significant market potential.

CANbridge owns global rights for **8** of the **14** drug assets


	Candidate	Mechanism	Discovery	IND-enabling	Ph 1	Ph 2/3	NDA	Marketed	Dev Strategy	Partner	Commercial Rights
Rare Onc.	 CAN008 (Asunercept)	CD95-Fc fusion protein	Glioblastoma Multiforme							 apogenix	Greater China
Rare Disease	 Hunterase® (Idursulfase beta)	ERT IDS	Hunter Syndrome (Mucopolysaccharidosis Type II)						In China for China	 GCPharma	Greater China
	 CAN 108 (Livmarli®)	IBAT inhibitor	China NDA Filed							 mirum	Greater China
			Alagille Syndrome (US / EU)								
			Progressive Familial Intrahepatic Cholestasis								
			Biliary Atresia								
	 CAN 106	Anti-C5 mAb	Paroxysmal Nocturnal Hemoglobinuria						In China for Global	 WuXi Biologics / Privus	Global
	 CAN 103	ERT GBA	Gaucher Disease							 WuXi Biologics	Global
	 CAN 107	Anti-FGF23 mAb	XLH							 WuXi Biologics / Privus	Global
	 CAN 104	ERT GLA	Fabry Disease							 WuXi Biologics	Global
	 CAN 105	Anti-Factor IXa/X bsAb	Hemophilia A						In China for China	 WuXi Biologics	Greater China
	 CAN 201	AAV sL65 GLA	Fabry Disease						Global for Global	 AstraZeneca LogicBio	Global
	 CAN 202	AAV sL65 GAA	Pompe Disease							 AstraZeneca LogicBio	Global
	 CAN 203	AAV9 SMN1	SMA							 UMass Chan Medical School	Global
	 Undisclosed	AAV	DMD							 UW Medicine Scriptr	Global
Other Onc.	 Caphosol™	Calcium phosphate rinse	Oral Mucositis							 EUSA Pharma	China
	 Nerlynx® (Neratinib)	Tyrosine kinase inhibitor	HER2+ Breast Cancer							 Pierre Fabre	Hong Kong, Taiwan, Macau



 Clinical trials performed by license partner
  Biologic
  Small Molecule
  Gene Therapy
  Medical Device

Pipeline Targets Diseases with \$15 Billion Potential

De-risked global pipeline with multiple programs in therapeutics with clinically validated MoAs

Commercial Rights	Pipeline	Indications	Prevalence		Global Sales
China	Hunterase®	MPS II	🇨🇳	8k	\$ >500 M
	CAN108	Alagille Syndrome	🇨🇳	10k	\$ 75 M
		PFIC	🇨🇳	5k	\$ >25 M
		Biliary Atresia	🇨🇳	50k	\$ NA
	CAN008	GBM	🇨🇳	55k	\$ NA
Global	CAN106	PNH	🇨🇳 23k	🌐 124k	\$ >5 B
		aHUS	🇨🇳 10k	🌐 32k	
		gMG	🇨🇳 234k	🌐 1,290k	
		NMOSD	🇨🇳 55k	🌐 171k	
	CAN 203	SMA	🇨🇳 14k	🌐 78k	\$ 1.4 B
	CAN103	Gaucher Disease	🌐 78k		\$ >1.5 B
	CAN104 CAN201	Fabry Disease	🌐 1,789k		\$ ~2 B
	CAN202	Pompe Disease	🌐 170k		\$ >1 B
	CAN107	XLH	🌐 117k		\$ ~1 B
	CAN105	Hemophilia A	🌐 340k		\$ ~4 B

 2022 Global Sales (US\$)

  2022 Global / China Prevalence

Abbreviations: GBM – Glioblastoma Multiforme; MPS II – Mucopolysaccharidosis type II; ALGS – Alagille Syndrome; PFIC – Progressive Familial Intrahepatic Cholestasis; PNH – Paroxysmal nocturnal hemoglobinuria; aHUS – Atypical Hemolytic Uremic Syndrome; gMG – Generalized Myasthenia Gravis; NMOSD – Neuromyelitis Optica Spectrum Disorders; XLH – X-linked hypophosphatemia; PD – Pompe Disease. Source: Frost & Sullivan and CANbridge Analysis, NCBI research, Endocrine Journal research, World Federation of Hemophilia research

Notes: CAN008 currently has no commercialized comparable product.

CANbridge Today

Well-positioned to Deliver Multiple Commercial and Development Milestones in Rare Diseases

1 Marketed Rare Disease Product

Hunterase



4 Late-stage Clinical Programs

Biliary Atresia

LIVMARLI

PNH

CAN106

Gaucher Disease

CAN103

Glioblastoma

CAN008

2 Anticipated Registration Filing/Approval in Next 12 Months

ALGS

LIVMARLI

PFIC

LIVMARLI

Business Highlights in 2022

Corporate and Business Development

RMB
79.0M

01

FY2022 net revenue **RMB 79.0M**, mainly attributable to sales from Hunterase in mainland China and Nerlynx in HK/TW

02

Announced license from UMass Chan Medical for the global development and commercialization rights to a novel second generation gene therapy for SMA

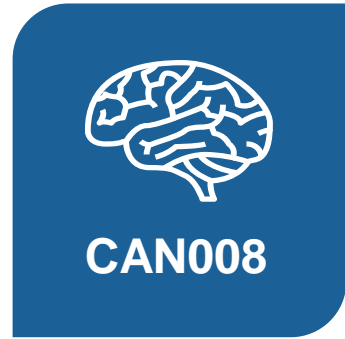
scAAV9



03

Obtained non-exclusive worldwide rights to LogicBio proprietary manufacturing process for Fabry and Pompe gene therapies in H2 2022. Completed full technology transfer from LogicBio by the end of 2022

Business Highlights in 2022



- Completed patient enrollment of the Ph2 trial in newly diagnosed GBM in Q1 2023
- Long-term data from phase 1/2 trial presented in ESMO in Mar 2023 showed 67% five-year OS rate vs. 8.2% in institutional database; 17.95 months median PFS vs. 5.8 months PFS in historical group



- First patient dosed in Phase 2 EMBARK study in BA in China
- Approved for treatment of ALGS under Early and Pilot Implementation Policy in Boao Lecheng International Medical tourism Pilot Zone
- has been granted priority review by the NMPA with a potential approval in H1 2023



- Dosed the first PNH patient in Phase 1b/2 trial
- Presented Ph1 SAD study data at European Hematology Association 2022 Congress and 3 other medical conferences



- Initiated Phase 1/2 trial in adult and adolescent Gaucher disease patients
- Initiated dosing in Part B (Ph2) in Q1 2023



- Presented pre-clinical data showing potential superiority against current marketed gene therapy at 2022 ASGCT/ESGCT/World Muscle Society

Part

02

Pipeline Update

Hunterase: The First and the Only Approved Enzyme Replacement Therapy for MPS II in China



Hunterase® – Early Commercialization In Non-reimbursed Market

Identification of new patients accelerates and
commercial insurance coverage expands

Mucopolysaccharidosis Type II (MPS II)



MPS II is a **rare, disabling**
and **life-threatening** genetic
disease



In **East Asian** countries, MPS II
is the most common form of
MPS disorders. Est. >3,000
patients in China



Chinese government has
included MPS II on the
“**First National List of
Rare Disease**” as a
disease group to target

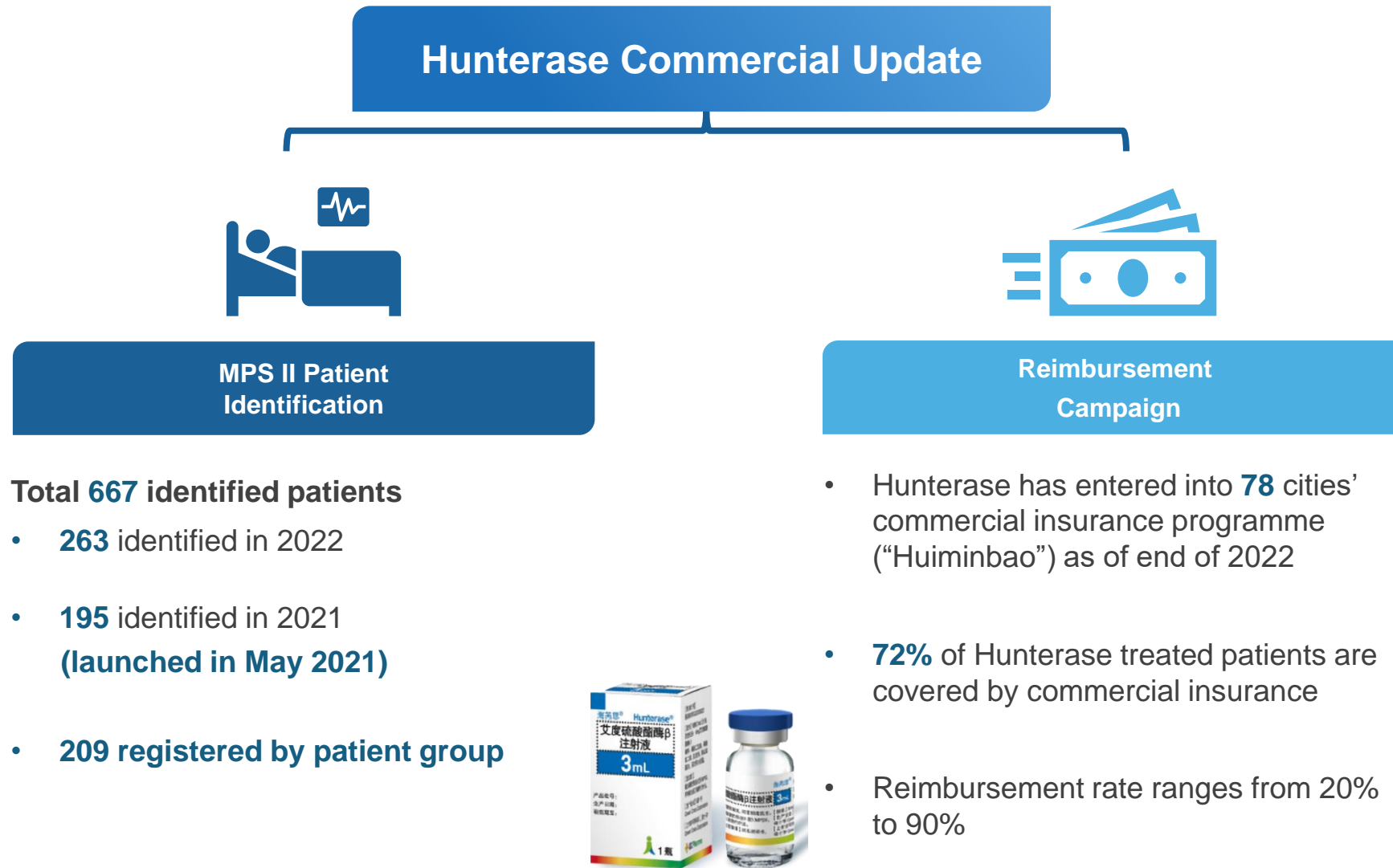


Life expectancy of patients
with severe MPS II (**60%-
80%** of cases) is significantly
reduced



Death occurs
generally before the
age of **25**

Hunterase® – 667 MPS II Patients Identified in China as of Dec 2022



First and only ERT treatment for MPS II in China

Livmarli® (CAN108) :

Near-term Launch in Rare Cholestatic Liver Diseases in China



CAN108 – IBAT Inhibitor for Rare Cholestatic Liver Diseases

A novel, oral, minimally-absorbed agent designed to selectively inhibit IBAT in the ileum and treat rare cholestatic liver diseases, including ALGS, PFIC and BA

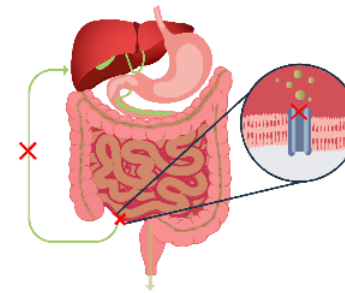
Recent Highlights

- Filed NDA to **China NMPA** and **Taiwan FDA** for **ALGS**, with estimated approval dates H1 2023 in mainland China and H2 2023 in Taiwan
- Mirum realized **\$75.1 million** in LIVMARLI (maralixibat) net product sales in the first full fiscal year of its U.S. launch
- Mirum dosed first patient in Phase 2 BA China study and reported positive topline **Phase 3 PFIC data and** label expansion for ALGS to include infants of 3 months+

Disease Overview

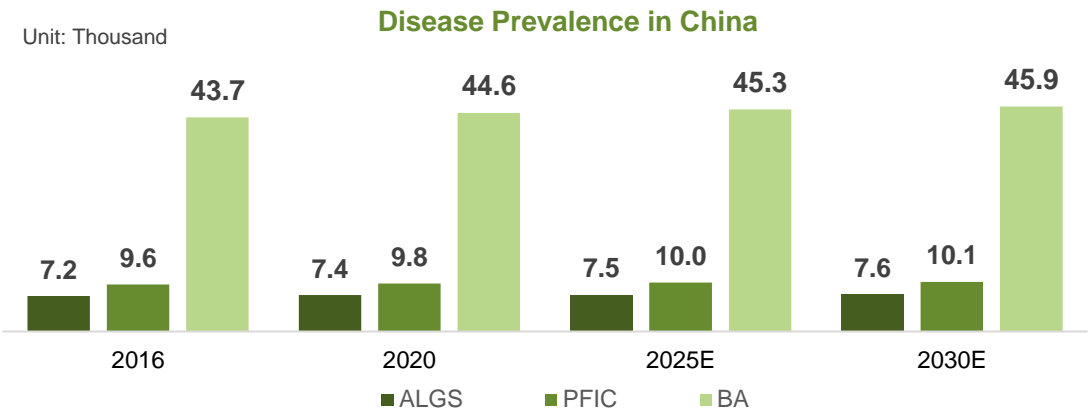
- Alagille Syndrome (ALGS):** a rare genetic disorder that can affect multiple organ systems of the body, including the liver, heart, skeleton, eyes and kidneys. No procedure to cure ALGS completely
- Progressive Familial Intrahepatic Cholestasis (PFIC):** a rare genetic liver disorder in which liver cells don't release bile properly, causing bile accumulation in the cells. Surgical treatment includes external or internal biliary diversions
- Biliary Atresia (BA):** a rare disease of the liver and bile ducts that occurs in infants. Currently no cure for BA available. Treatments include liver transplant and Kasai procedure

Mechanism of Action



- IBAT is primarily responsible for recycling bile acids from the intestine ileum back to the liver
- Elevated bile acids damage the liver and lead to cholestatic liver disease
- CAN108 is designed to inhibit IBAT in the ileum and result in more bile acids being excreted in the feces, leading to lower levels of bile acids systemically, thereby reducing bile acid mediated effects and liver damage

Epidemiology



Source: Frost & Sullivan Analysis. Abbreviations: IBAT, ileal sodium-dependent bile acid transporter



In China for China

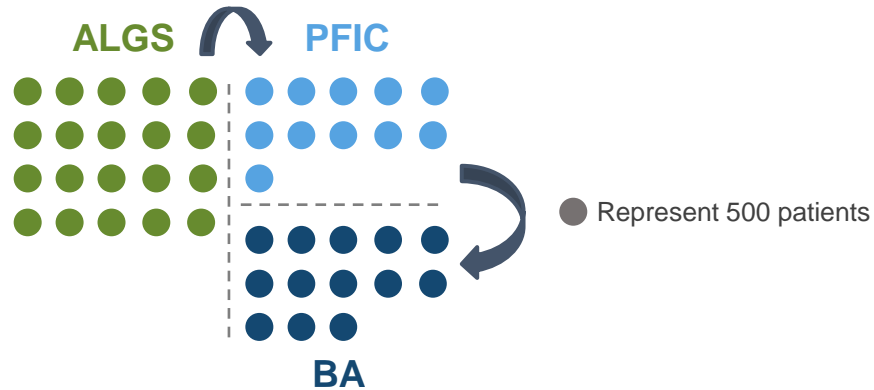
In China for Global

In Global for Global

CAN108 – Commercialization Strategy

Large and robust safety dataset provides strong support
for further studies in BA

~22,000 targeted patients with ALGS, PFIC, BA in China



Next
Catalyst ➔

Key Development Timeline

Potential ALGS CN approval
in H1 2023

Potential ALGS TW approval
in H2 2023

Potential ALGS HK approval
in Q1 2024

Potential PFIC CN/TW
approval
in H2 2024

Growth Opportunities:

- Priority review granted by NMPA with potential approval in H1 2023
- Anticipated commercial launch for PFIC in 2024
- Phase 2 BA EMBARK trial is ongoing



In China for China

In China for Global

In Global for Global

CAN106: Pipeline in A Product



CAN106 – Long-acting Anti-C5 Therapy for Complement Disorders

Significant unmet need in treating patients
with complement-related disease in China and across the globe

Recent Highlights

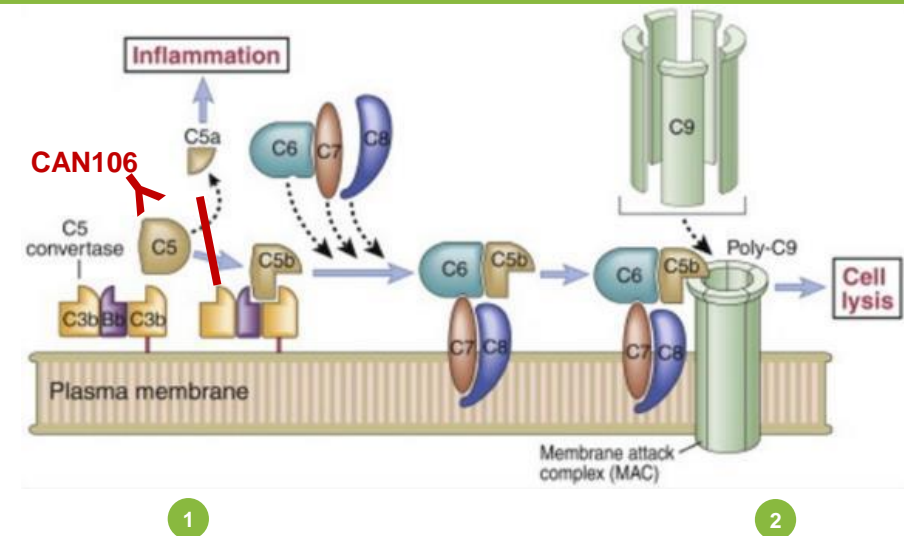
Obtained **global rights to develop, manufacture and commercialize** CAN106 through a strategic agreement with WuXi Biologics and Privus (Originator)

Currently in Phase 1b/2 study in patients with PNH in China (first PNH patient dosed in March 2022)

Data of Phase 1 SAD study presented at EHA 2022. **Safe and well-tolerated** with mostly mild or moderate adverse events and no drug-related serious adverse events

Obtained **Orphan Drug Designation** for the treatment of MG (Nov 2022)

Mechanism of Action



CAN106 binds to the α chain of C5, which prevents C5 from being cleaved into C5a and C5b by C5 convertase, thus preventing MAC formation and cell lysis

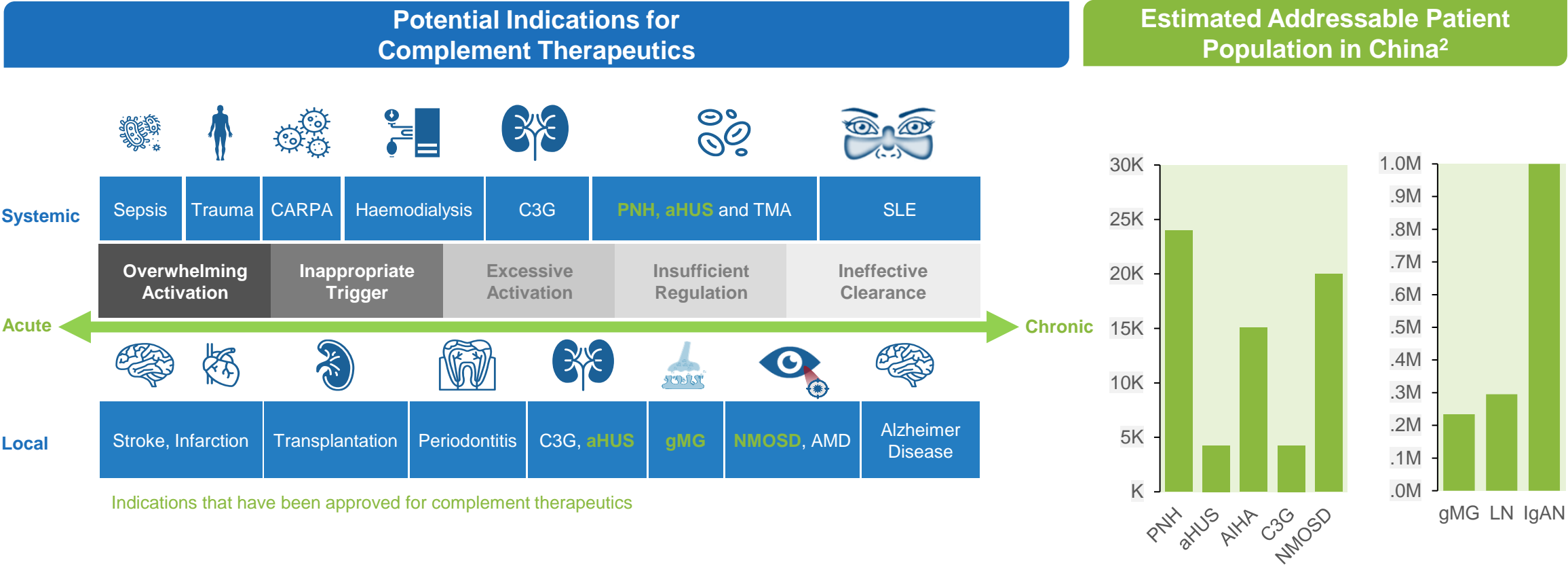
CAN106 preserves the generation of C3b, which is essential for the clearance of circulating immune complexes and the normal phagocytosis of bacterial and fungal pathogens



CAN106 – Long-acting Anti-C5 Therapy for Complement Disorders

Potential “Pipeline in a Product”. Initial development efforts focused on PNH treatment.

Estimated global market revenue projections of >\$9B in 2025¹



Abbreviation: PNH, paroxysmal nocturnal hemoglobinuria. Notes: 1. According to Alexion Investor Day 2020 news release published on October 6, 2020. 2. Risitanon and Rotoli, 2008 & Chinese KOL interview; China aHUS Diagnosis and Treatment Consensus, 2017; China MG Diagnosis and Treatment Guideline, 2015 & Howard et al, 2017; Zanella and Barcellini, 2014 & Berentsen and Sundic, 2015; Mahmoud et al, 2016; CANbridge research

CAN106 – Complement Advisory Board

Board will offer guidance on the CAN106 global development program,
as well as explore the potential for CAN106 in other indications



**Anthony Amato,
MD**

- Brigham and Women's Hospital Distinguished Chair in Neurology
- Chief of the Neuromuscular Division and Director of the Clinical Neurophysiology Laboratory at Brigham and Women's Hospital
- Professor of Neurology at Harvard Medical School



**Robert Colvin,
MD**

- Pathologist-in-Chief, Emeritus at Massachusetts General Hospital
- The Benjamin Castleman Distinguished Professor of Pathology at Harvard Medical School



**Gerald Cox, MD,
PhD**

- Chief Development Strategist & Interim Chief Medical Officer
- Clinical geneticist and pediatrician at Boston Children's Hospital
- Former Chief Medical Officer at Editas Medicine
- Vice President, Rare Disease Clinical Development at Sanofi



**Jean Francis,
MD**

- Medical Director of Kidney Transplant Program at Boston Medical Center and Boston Univ Sch of Med
- Medical Director of Combined Pancreas Transplant Program at Boston Medical Center and Brigham and Brigham & Women's Hospital
- Associate Professor of Medicine at Boston Univ Sch of Med



Richard Polis, MD, MHSc

- Clinical Development Consultant
- Former CMO at Artax Biopharma
- Senior VP and Head of Transl Med at Sanofi-Genzyme R&D Center
- Clin Dir of Arthritis Unit and Chair of Mallinckrodt Clin Res Unit Scientific Adv Comm at Massachusetts General Hospital
- Associate Professor of Medicine at Harvard Medical School



**Sushrut Waikar,
MD, MPH**

- Chief of Nephrology at Boston Medical Center
- Norman G. Levinsky Professor of Medicine at Boston University School of Medicine
- Former Constantine L. Hampers, MD Distinguished Chair in Renal Medicine at Brigham and Women's Hospital



Brian Weinshenker, MD

- Professor of Neurology at University of Virginia
- Former Professor of Neurology at Mayo Clinic

**Neuromuscular
Disorders**

**Immunopathology of
Kidney Disease and
Organ Transplant
Rejection**

**Rare Disease Drug
Development**

**Organ Transplant, PNH,
Thrombotic
Microangiopathy**

**Rare Disease Drug
Development,
Rheumatologic Diseases**

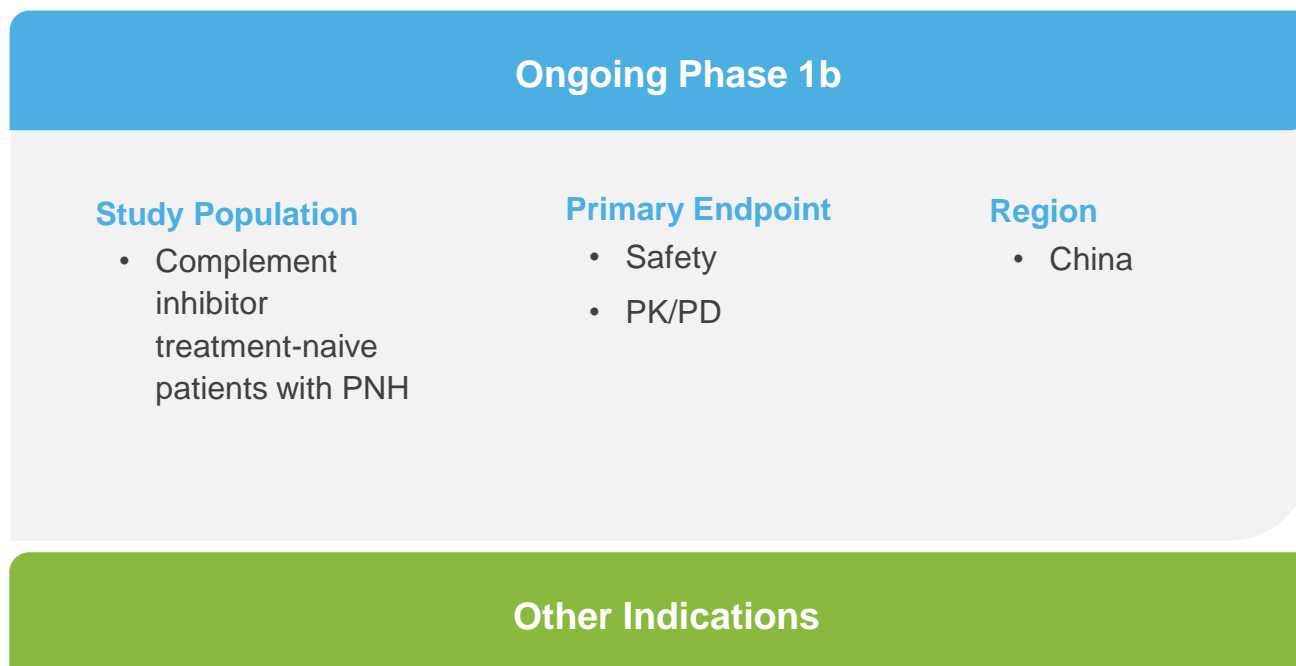
**Renal Diseases, Non-
invasive Biomarkers of
Renal Injury and Fibrosis**

**NMOSD and Other CNS
Demyelinating Diseases**



CAN106 – Optimizing Commercial Pathway in China

Phase 1b/2 Open-label, Multiple Ascending Dose Study to Evaluate CAN106
in Complement Inhibitor Treatment-Naive Patients with PNH



Preparation of other programs will be initiated in 2H 2023

Indications under considerations include: gMG, NMOSD, aHUS, etc.

Next Catalyst ➔



CAN008: Development in Newly Diagnosed GBM



CAN008 – CD95-Fc Fusion Protein for Glioblastoma Multiforme (GBM)

CAN008 is in clinical development as a first-line therapy for GBM in China

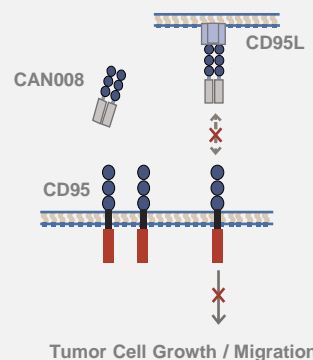
Recent Highlights

- Currently in Phase 2 registrational trial in newly diagnosed GBM in China. Patient enrollment (N = 117) completed in March 2023
- Long-term data from phase 1/2 trial presented in ESMO in Mar 2023 shows
 - 67% five-year OS** rate compared to 8.2% in institutional database
 - 83% OS** at two years vs. 34.3% OS from institutional database
 - 17.95 months median PFS** vs. 5.8 months PFS in historical group

GBM Overview

- A rare oncologic disease with **lower incidence** than other cancer types
- Median age of diagnosis is 64 years, and it is slightly more common (1.59 times) in men as compared to women
- The **most common and malignant** type of glioma in adults. About 45% of gliomas are glioblastoma multiforme
- Estimated **5-year survival of 5.5%** globally and below 5% in China
- Treatment options: surgical resection, adjuvant chemotherapy with TMZ, tumor treating field (TTF), bevacizumab (Avastin)

Mechanism of Action

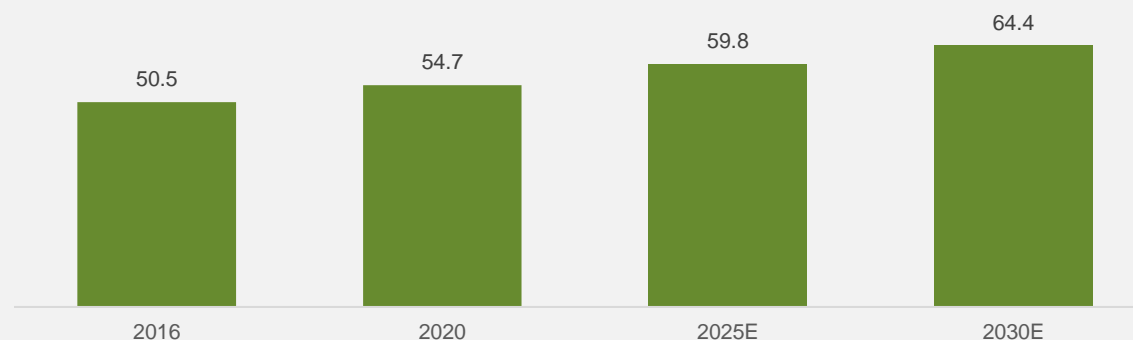


- CD95L binds to its receptor, CD95, on the cell surface and induces the oligomerization of CD95, which triggers an intracellular signaling cascade that stimulates tumor cell growth and migration
- CAN008 acts as a soluble receptor that specifically binds to CD95L and blocks the endogenous CD95 / CD95L signaling pathway in tumor cells. CAN008 also blocks CD95 / CD95L engagement on T cells, which prevents T cell apoptosis and restores immune function
- As the oligomerization of CD95 is blocked and signal transduction is inhibited, the growth and migration of tumor cells are suppressed

Epidemiology

Annual Incidence of GBM in China

Unit: Thousand



Source: Frost & Sullivan Analysis. Notes: GBM, glioblastoma multiforme; TMZ, temozolomide



In China for China

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CAN008 – Phase 1 in Newly Diagnosed GBM

CAN008 shows clear signs for clinical efficacy
in newly diagnosed GBM patients

Newly Diagnosed Glioblastoma (n = 10)

Low dose cohort (n=3)

Standard of care
(RT + TMZ)
+
CAN008
(weekly i.v. 200mg)
Up to 2 years

Standard dose cohort (n=7)

Standard of care
(RT + TMZ)
+
CAN008
(weekly i.v. 400mg)
Up to 2 years

Safety

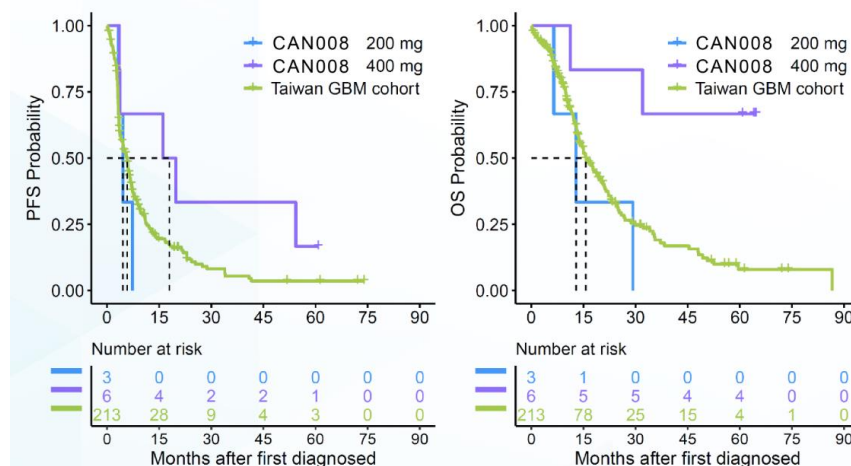
- **No specific safety issues** when CAN008 (200 or 400 mg) was combined with RT and TMZ.
- **No subjects discontinued** due to treatment-emergent adverse events.
- No patients experienced dose-limiting toxicity (DLT).
- Maximum administered dose of **400 mg IV once weekly** recommended as the RP2D.

Source: Wei K-C et al, Sci Rep 2021;11:24067

Efficacy

PFS rates	200 mg cohort	400 mg cohort
PFS-3 months	33.33%	71.42%
PFS-6 months	33.33%	57.14%
PFS-9 months	0% (all progressed)	57.14%
PFS-12 months	-	57.14%
Median PFS	2.37 months	N/A ⁽¹⁾

Kaplan-Meier survival curves of the historical GBM cohort and CAN008 cohorts with different dosages



Long-term follow-up
presented at 2023
ESMO: 67% survival
rate at five years



In China for China

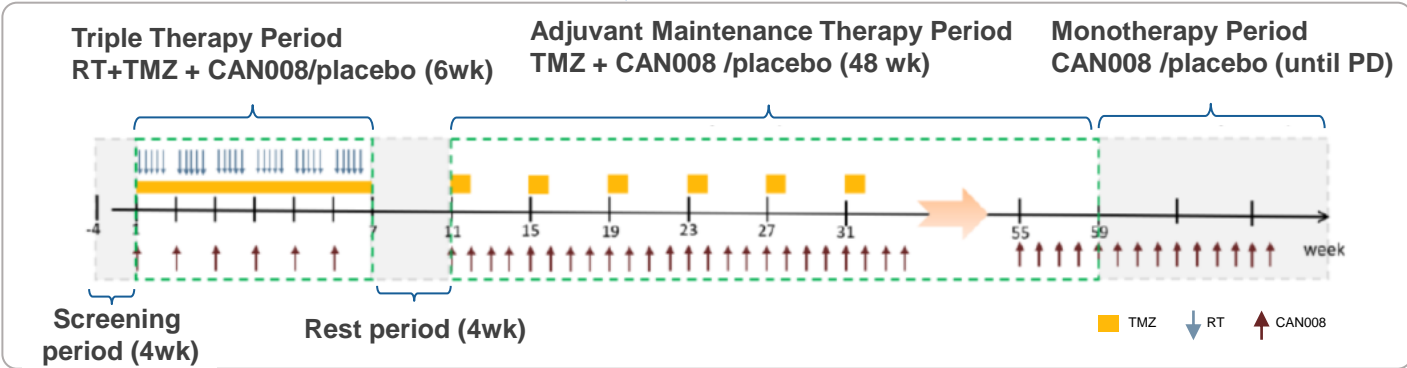
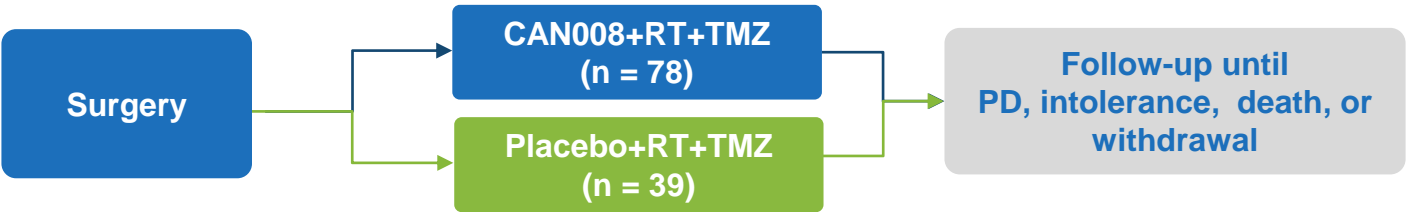
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CAN008 – Ongoing Phase 2 Registrational Trial in Newly Diagnosed Glioblastoma

Phase 2 Multi-center, randomized,
double-blind, placebo-controlled study



Primary endpoint

- Progression-free survival (PFS)

Interim Readout

- Progression of 37 cases

Next
Catalyst



CAN103:

The First Enzyme
Replacement
Therapy For Gaucher
Disease Developed
In China

 CANbridge



CAN103 – Enzyme Replacement Therapy for Gaucher Disease

Aspired to provide the first domestically manufactured ERT
for Gaucher patients in China

CAN103 Overview



First ERT for
Gaucher Disease
(GD) developed in
China



Company holds
global proprietary
rights to develop
and commercialize



GD inherited in
autosomal recessive
manner; common
symptoms include
hepatosplenomegaly,
anemia, bone disease in
addition to neuronopathy



One of the best
known and
prototypical rare
diseases in China,
approximately **6,000**
patients



Included in the
**“First National
List of Rare
Disease”**

**Next
catalyst** ➔

Key Development Timeline

Phase 1 FPD
in July 2022

Phase 2 FPD
in Q1 2023

Registration filing
in **Mid 2024**



CAN203: Next-gen Gene Therapy for SMA

CAN203 – Gene Therapy for Spinal Muscular Atrophy

Pathophysiology Illustration

- SMA is characterized by dysfunction of α -motor neurons that under healthy conditions innervate skeletal muscles and are responsible for muscle contraction
- In SMA patients, defects in SMN1 gene result in decreased SMN protein expression
- Decreased SMN expression leads to dysfunction/loss of α -motor neurons
- Dysfunction/loss of α -motor neurons leads to muscle atrophy and weakness

Epidemiology

- Autosomal recessive genetic inheritance
- 1 in 6,000 to 1 in 10,000 children born with SMA
- Affects all racial and ethnic groups

Unmet Need

- In older SMA population for which the first-generation gene therapy Zolgensma is not indicated
- Black box warning of serious liver injury associated with Zolgensma
- Access limitation due to high price

Source: Cowen equity research and SMA foundation.

CAN203 Gene Therapy Design

- **Second-Gen gene therapy to potentially treat SMA Type 1-3**
- **Similar capsid as used in SOC**
- **Endogenous promoter: controlled, targeted tissue expression to avoid unwanted toxicity in liver and heart**
- **Codon optimized: enhanced tissue expression**

Key Development Timeline

Next catalyst



H1 2023

Additional preclinical data to present at a medical conference

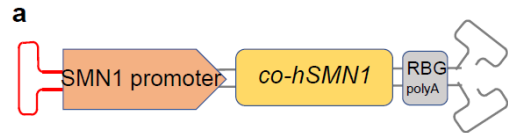
Q4 2024
Global IND



CAN203 – Preclinical Data Presented at 2022 ASGCT

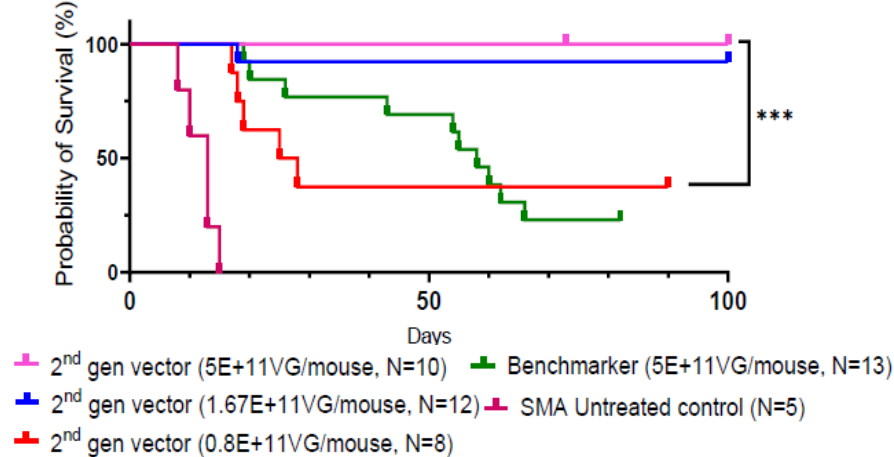
Head-to-head comparison with our 2nd gen vector and a benchmark vector, whose design is similar to the one used in Zolgensma®, demonstrated therapeutic advantages over the benchmark vector

2nd Gen Vector: Endogenous SMN1 promoter isolated and inserted upstream of the codon-optimized hSMN1

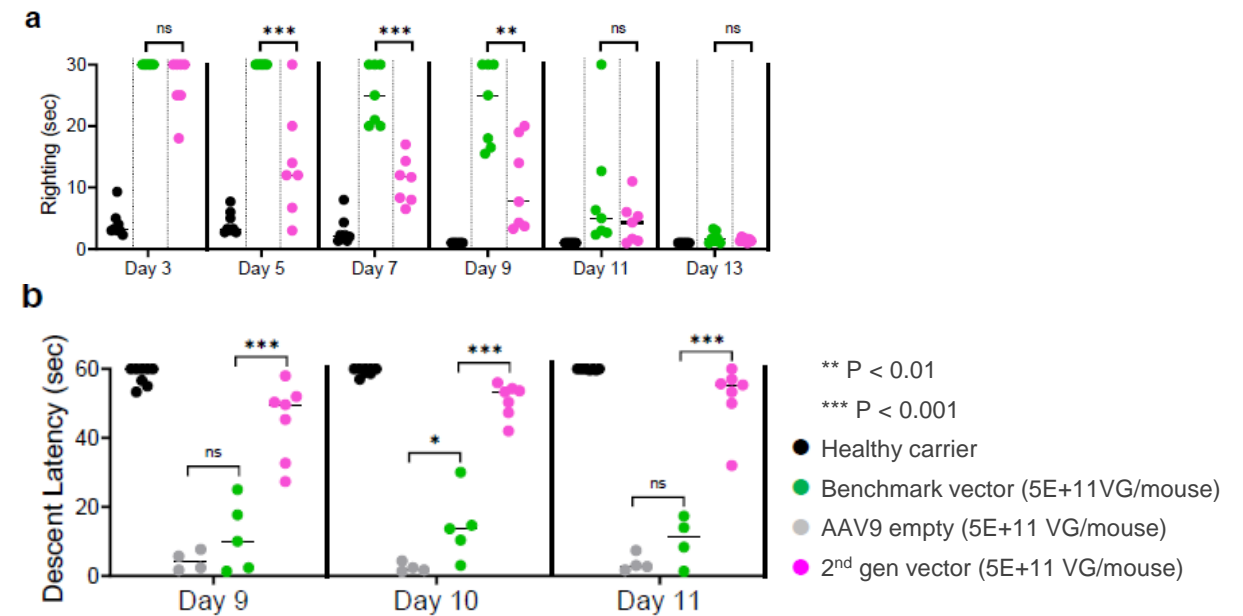


pAAVsc-SMNp-co-hSMN1 (2nd gen vector)

Survival curve of vector-treated mice



2nd gene vector conferred significantly better restoration of muscle function the benchmark vector in SMA mice



In vivo data 2nd gen vector demonstrate advantages in extension of life span, elimination of liver toxicity, and improved restoration of muscle function

Source: <https://annualmeeting.asgct.org/abstracts/abstract-details?abstractId=6140>

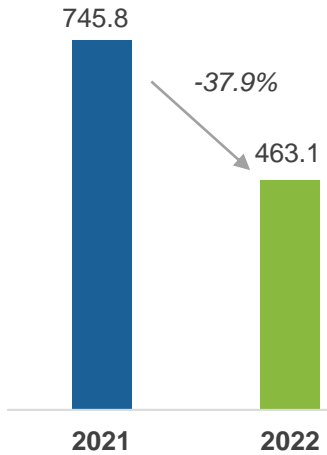
Part

03

Financial Review

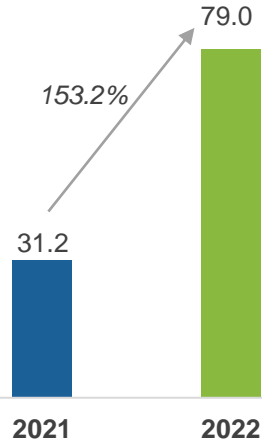
2022 Financial Highlights

RMB Million



Cash Balance

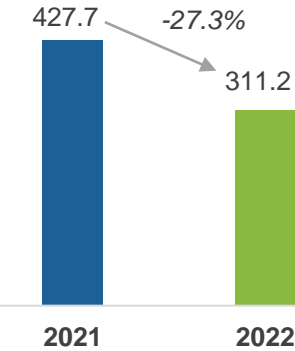
YoY decrease of RMB 282.7M, primarily attributed to net cash outflows used in operations



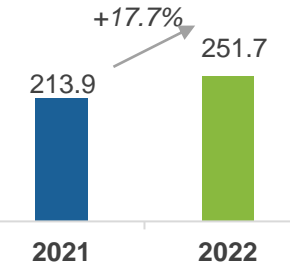
Revenue

YoY increase of RMB 47.8M mainly attributable to the increase of sales from Hunterase® and Nerlynx®

R&D expenses including milestones payment

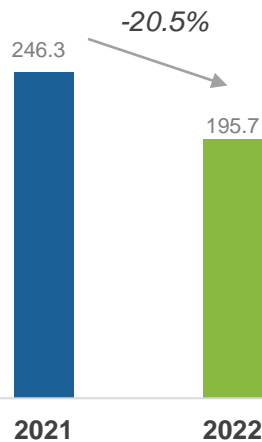


R&D expenses excluding milestones payment



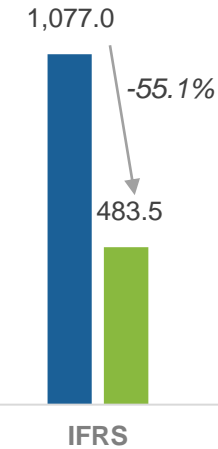
R&D Expenses

YoY decrease of RMB 116.5M primarily attributable to decreased license fees which was partially offset by increased testing and clinical trial expenses



SG&A

YoY decrease of RMB 50.6M, such decrease was primarily attributable to the decrease in our listing expenses and professional fees. And such decrease was also due to the decrease in employee costs and marketing expenses as a result of the reduction of marketing activities during the year 2022 and due to the increased effectiveness in sales activities during the year of 2022.



Loss for the Year

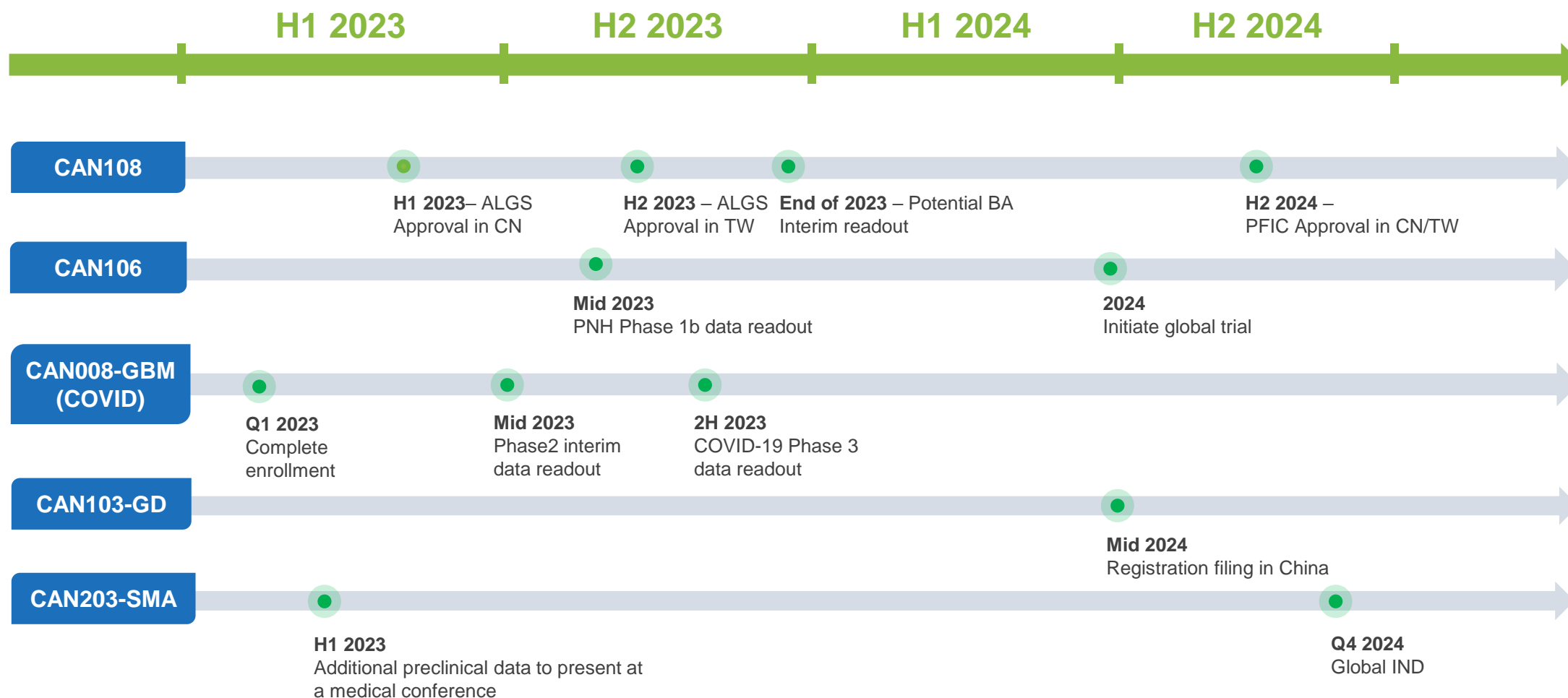
Adjustments to IFRS measure was driven by (i) a one-time, non-cash, IFRS fair value changes of our pre-IPO convertible redeemable preferred shares and derivative financial instruments, (ii) the share-based payment expenses, and (iii) the listing expense

Part

04

Outlook

Multiple Catalysts to Build Valuation into 2023 and Beyond



Q&A

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THANK YOU



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WeChat Official Account

Part 05 Appendix



Income Statement

RMB'000	Year ended December 31	
	2022	2021
Revenue	78,972	31,161
Cost of sales	(30,078)	(12,385)
Gross profit	48,894	18,776
Other income and gains	12,883	13,402
Selling and distribution expenses	(86,782)	(100,748)
Administrative expenses	(108,907)	(145,517)
Research and development expenses	(311,174)	(427,658)
Fair value changes of convertible redeemable preferred shares	-	(462,436)
Fair value changes of derivative financial instruments	-	34,454
Other expenses	(31,526)	(4,200)
Finance costs	(6,863)	(3,079)
Loss before tax (IFRS Measure)	(483,475)	(1,077,006)
Adjustments to Non-IFRS measure	26,822	495,684
Adjusted loss for the period* (Non-IFRS Measure)	(456,653)	(581,322)

Balance Sheet

	December 31	
RMB'000	2022	2021
Property, plant and equipment	15,003	9,564
Right-of-use assets	129,714	19,978
Intangible assets	49,011	51,269
Other non-current assets	3,157	-
Total Non-current Assets	196,885	80,811
Inventories	9,824	13,448
Trade receivables	19,054	9,141
Prepayments, other receivables and other assets	13,175	43,307
Cash and bank balances	463,107	745,815
Total Current Assets	505,160	811,711
Trade payables	107,540	43,607
Other payables and accruals	130,670	103,423
Interest-bearing bank and other borrowings	26,867	30,868
Lease liabilities	13,028	7,882
Total Current Liabilities	278,105	185,780
Interest-bearing bank and other borrowings	10,779	-
Lease liabilities	104,606	13,351
Total Non-current Liabilities	115,385	13,351
Total Equity	308,555	693,391