CANbridge Pharmaceuticals

Corporate Presentation

November 2022

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Our Vision

To be a **Global Biopharmaceutical Company**
Delivering **Life-changing Therapies to Patients**
Built Upon **a Foundation in China**

01

Business Overview

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

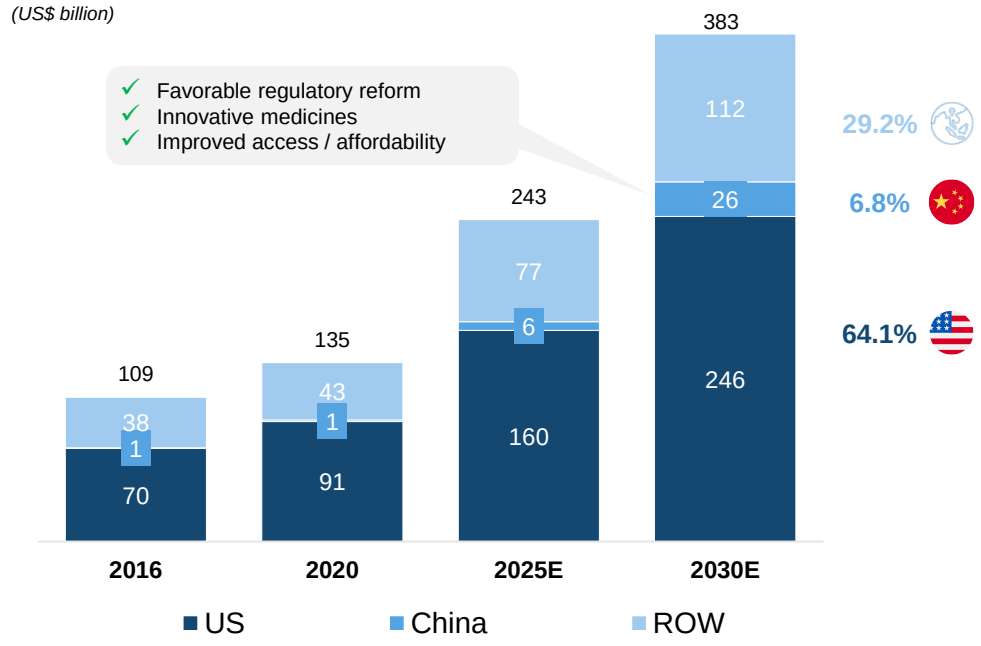
A rare disease pioneer dedicated to addressing vast and unmet medical needs

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

Significant Global Opportunity Targeting Rare Diseases

Proven large global rare disease markets. China represents potentially biggest untapped market

Global Rare Disease Drug Market[1] (By Region)

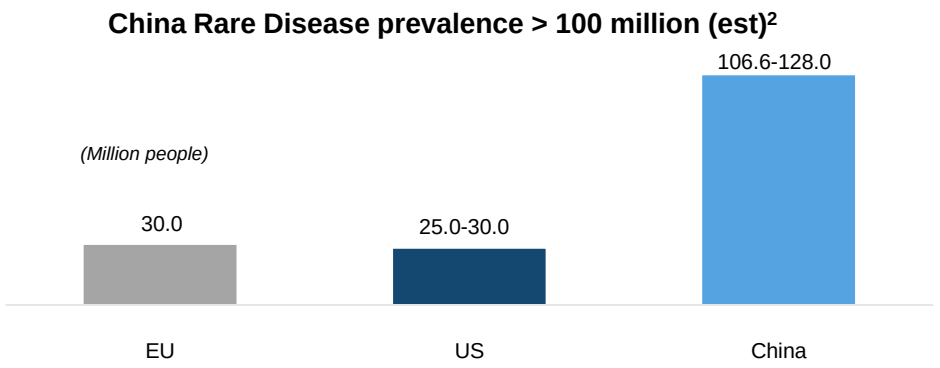


China's contribution to global rare disease market

2020
US$ 1.3 Bn
<1%

2030E
US$ 25.9 Bn
6.8%

Rare Disease Prevalence in U.S., EU & China (est.)



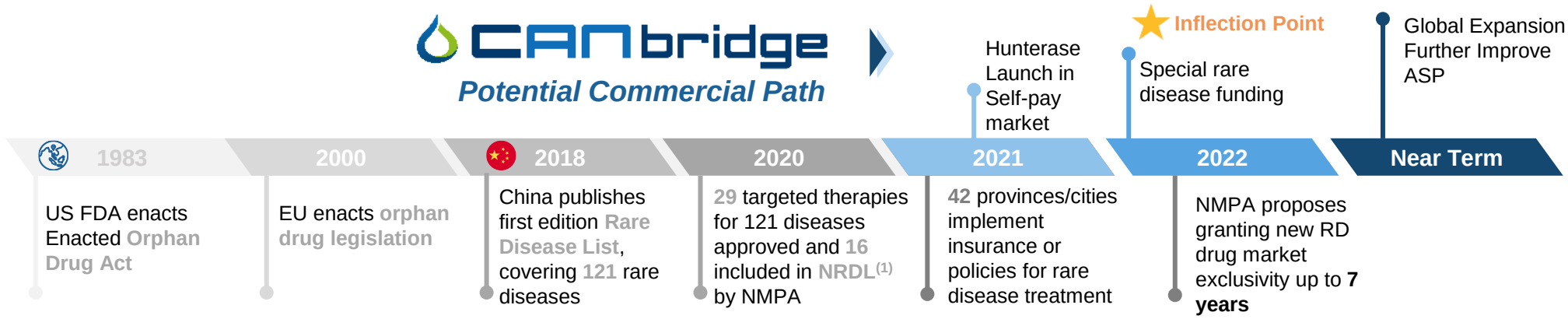
Global Competitors' Strong Rare Disease Drugs Sales

The ten top-selling orphan drugs achieved a combined sales of US $42.8 billion globally in 2020

Source: Frost & Sullivan Analysis. Notes: 1, Rare disease drugs in this market include drugs indicated for rare disease only and drugs obtaining orphan drug designation first but their indication expanded later of which generated most of their revenue, such as Humira. However, the part of revenue generated by expanded non-orphan indications was not included in this market. 2, It is an appropriately reasonable estimate that China may have a total prevalence base which exceeds 100 million patients suffering from one or more forms of rare diseases, by way of extrapolation adopting the broader definition in the U.S. or Europe.

A Rapidly Evolving Market for Developing Rare Diseases Products

Emerging Favorable Regulatory Framework



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**

Financial incentives:

- New China plan to slash the **Value Added Tax (VAT) by 80%** on select disease therapies and active pharmaceutical ingredients

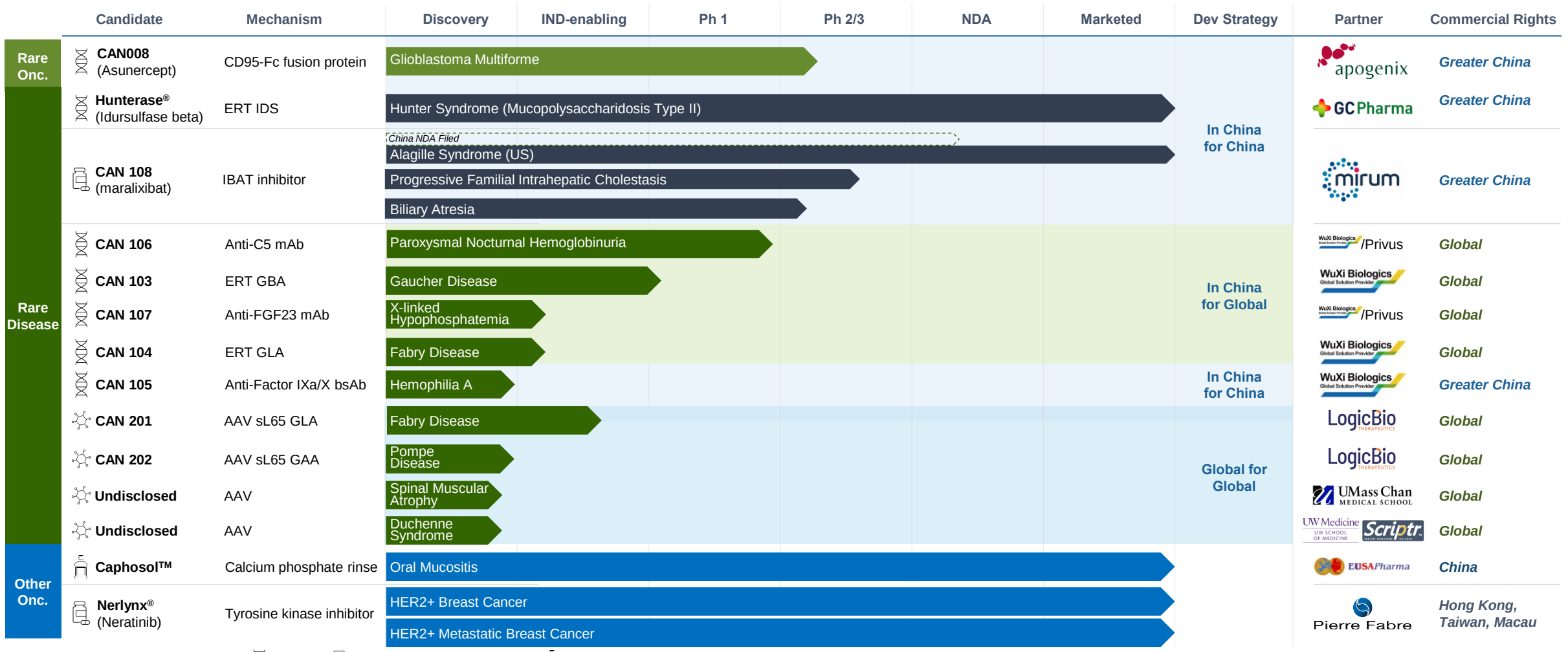
Increased awareness:

- 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

Source: Frost & Sullivan Analysis. Notes: 1, NRDL, National Reimbursement Drug List in China; 2, as of December 2020

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 **7** of the **13** drug assets



Developing a Gene Therapy Portfolio with Potential Best-in-Class Global Assets

Gene therapy holds the promise to transform treatments for LSDs and neuromuscular diseases from ameliorative to curative

In-licensed Gene Therapy Programs and In-house Tech Platform Pipeline

Candidate	Discovery	IND-Enabling	Clinical	Collaborator
CAN201	Fabry		Exp. 2024	LogicBio Therapeutics
CAN202	Pompe		Exp. 2023	LogicBio Therapeutics
Undisclosed	Spinal Muscular Atrophy			UMass Chan Medical School
Undisclosed	Duchenne Muscular Dystrophy			UW Medicine UW School of Medicine / Scriptr
CANbridge Innovative Platform	Tissue-Specific Targeting Platform	PoC Exp. 2022-2023	Protentional indication 1 Protentional indication 2 Protentional indication 3	CANbridge In-house

Upcoming milestones

US R&D Center. Burlington, MA

- 24,500 sq. ft. (up to 90 FTEs)
- AAV process lab (up to 50L scale)
- AAV analytical lab
- Research discovery lab
- Opened in 2H 2022

2nd Generation Capsid and Transgene engineering

- **LogicBio**: Novel sAAVy capsid (sL65) with improved functional transduction and immunological profile compared to LK03
- **UMass**: CNS and muscle tropic new AAV
- **UW and Scriptr**: Dystrophin with improved function

CANbridge Innovative AAV Platform

- Using tissue specific cell surface receptors for targeting
- AAV platform enables future development in multiple CNS/muscle-related diseases
- Patent filing in process

Abbreviations: FTE, full-time equivalents

Pipeline Targets Diseases with Significant Revenue Potential

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

In China for China

	Prevalence	$ Global Sales
MPS II Hunterase®	8k	666
ALGS CAN108	10k	70*
PFIC CAN108	5k	24*
BA CAN108	50k	
GBM CAN008[1]	55k	

In China for Global

	Prevalence	$ Global Sales
PNH CAN106	124k	
aHUS CAN106	32k	
gMG CAN106	1,290k	
NMOSD CAN106	171k	
Four Indications		5,362
GD CAN103	78k	1,517
FD CAN104 CAN201	1,789k	1,972
PD CAN202	170k	1,225
XLH CAN107	117k	848
Hemophilia A CAN105	340k	3,818

Rare disease/ Rare Cancer $ 2021 Global Sales (US$ MM) 2021 Global / China Prevalence

Abbreviations: GBM – Glioblastoma Multiforme; MPS II – Mucopolysaccharidosis type II; ALGS – Alagille Syndrome; PFIC – Progressive Familial Intrahepatic Cholestasis; BA – Biliary Atresia; GD – Gaucher Disease; PNH – Paroxysmal nocturnal hemoglobinuria; aHUS – Atypical Hemolytic Uremic Syndrome; gMG – Generalized Myasthenia Gravis; NMOSD – Neuromyelitis Optica Spectrum Disorders; XLH – X-linked hypophosphatemia; FD – Fabry Disease; PD – Pompe Disease. Source: Frost & Sullivan and CANbridge Analysis, NCBI research, Endocrine Journal research, World Federation of Hemophilia research Notes: 1. CAN008 currently has no commercialized comparable product. * estimated 2022 sales

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

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

02

Pipeline Update

Highlights for Ongoing Clinical Programs in China

Alagille Syndrome

10,000 Patients

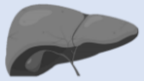
$ > 100 M*

CAN108

NDA Filed

- ***Anticipated CN approval and commercial launch in 2023***
- *Initiated patient community and education projects*
- *EAP ongoing in Hainan*

Progressive Familial Intrahepatic Cholestasis



5,000 Patients

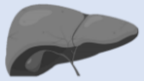
$ > 50 M*

CAN108

Phase 3 Completed^

- *Established registration strategy in mainland China, HK and TW*

Biliary Atresia



50,000 Patients

$ > 250 M*

CAN108

Phase 2 Ongoing

- *A higher prevalence observed in Asian than Caucasian infants*

Glioblastoma

55,000 Patients

$ >500 M*

CAN008

Phase 2 Ongoing

- *Devastating disease with OS less than 2 years*
- *No targeted therapy approved yet*
- *Studied in newly diagnosed patients*

Paroxysmal Nocturnal Hemoglobinuria

24,000 Patients

$ > 250 M*

CAN106

Phase 1/2 Ongoing

- *Patients have no access to complement therapy in China*

Gaucher Disease

3,000 Patients

$ > 50 M*

CAN103

Phase 1/2 Ongoing

- *CAN103 targets the root cause of the disease*

Source: Frost & Sullivan Analysis. * potential commercial opportunity in China, based on CANbridge estimates (million USD). ^ Phase 3 global study is carried out by partner Mirum

Hunterase® – The Only ERT Approved for MPS II Launched in China

Identification of new patients accelerates and commercial insurance coverage expands

Overview of 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
- Chinese government has included MPS II on the "**National Rare Disease List**" 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 Commercial Updates

MPS II Patient Identification

Total 539 identified patients

- **135** patients newly identified in 1H 2022;
- **195** identified in 2021 since launch in May 2021
- **209** registered by patient group

Reimbursement Campaign

- **5** provinces and **42** cities, Hunterase covered by commercial insurance
- **64%** of Hunterase treated patients are covered by commercial insurance

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

CAN108 Highlights

Obtained an **exclusive license** to develop, manufacture and commercialize Livmarli (maralixibat) in Greater China from Mirum

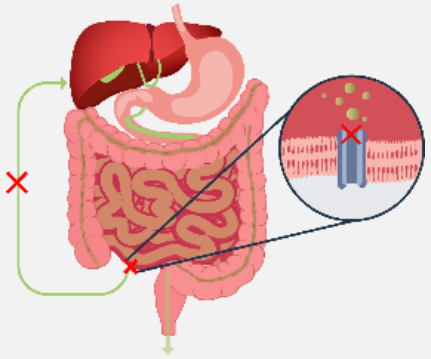
Approved to treat cholestatic pruritus in patients with **Alagille syndrome (ALGS)** who are aged 1 year or older in the U.S. in September 2021

Currently no approved product in China for **ALGS**, **PFIC** or **BA** (post-Kasai)

Extensive safety dataset; evaluated in **1,600+** human subjects and studied in completed and ongoing clinical trials for ALGS and PFIC with **120+** children

Potential **to improve long-term outcomes** liver transplant

Mechanism of Action



1. IBAT is primarily responsible for recycling bile acids from the intestine ileum back to the liver
2. Elevated bile acids damage the liver and lead to cholestatic liver disease
3. 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

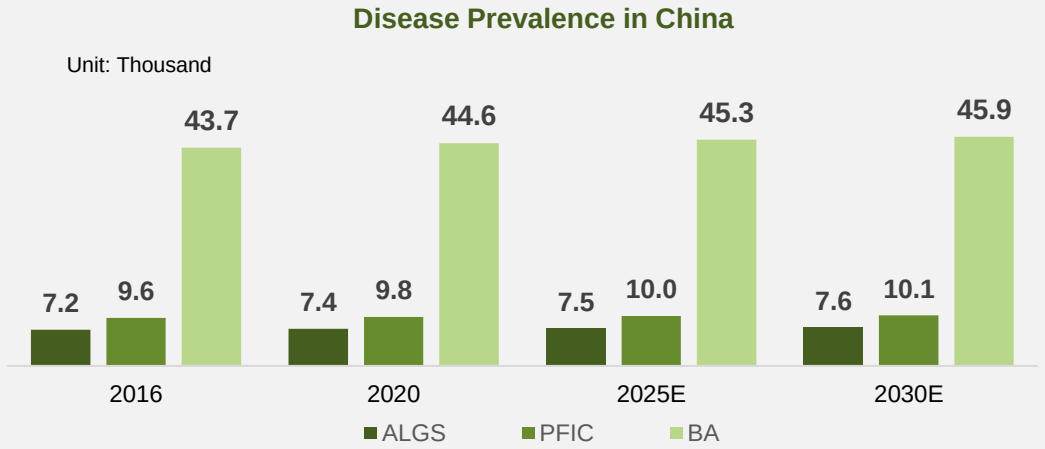
Disease Overview

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

Epidemiology

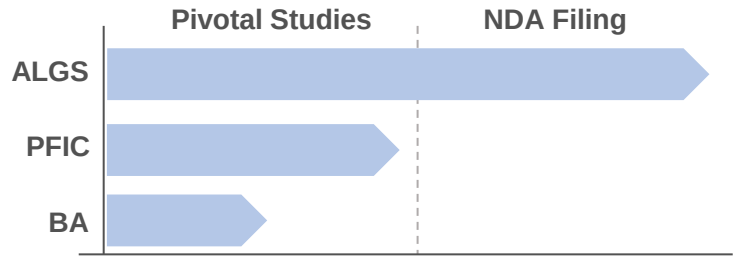


Source: Frost & Sullivan Analysis. Abbreviations: IBAT, ileal sodium-dependent bile acid transporter

CAN108 – Clinical Development Plan

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

CAN108 Development Status in China



ALGS

✓ NDA filed in mainland China and Taiwan

✓ EAP programs in mainland China and Hong Kong

PFIC

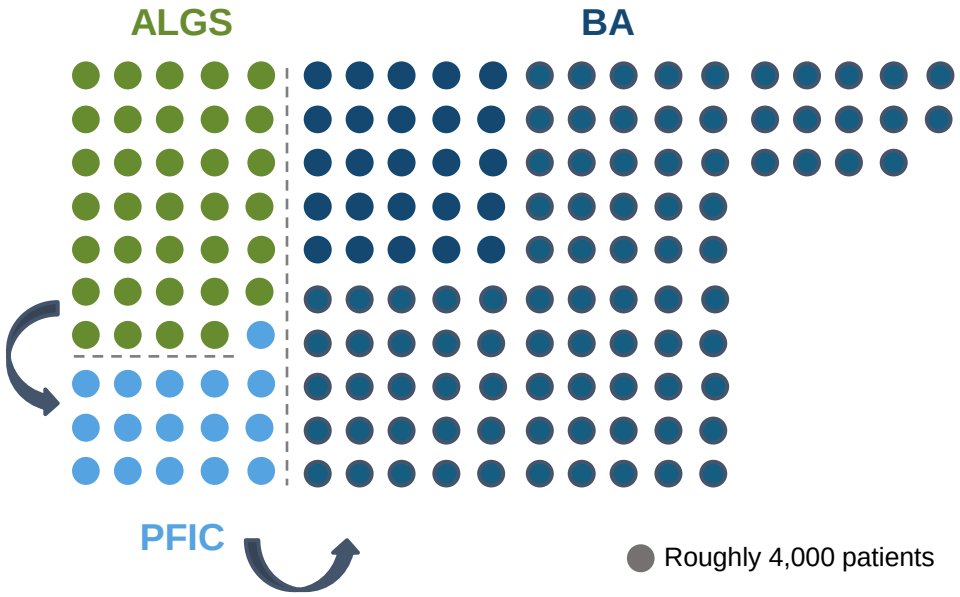
✓ To file China NDA after Mirum filing, potentially in early 2023

✓ Mirum announced positive topline phase 3 Data in Oct 2022

BA

✓ First patient dosed in Ph 2 China trial

>70,000 patients with ALGS, PFIC, and BA in China



Growth Opportunities:

- Anticipated commercial launch for ALGS in 2023 and PFIC in 2024

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

CAN106 Highlights

FDA Granted Orphan Drug Designation to CAN 106 for the Treatment of Myasthenia Gravis in Nov 2022

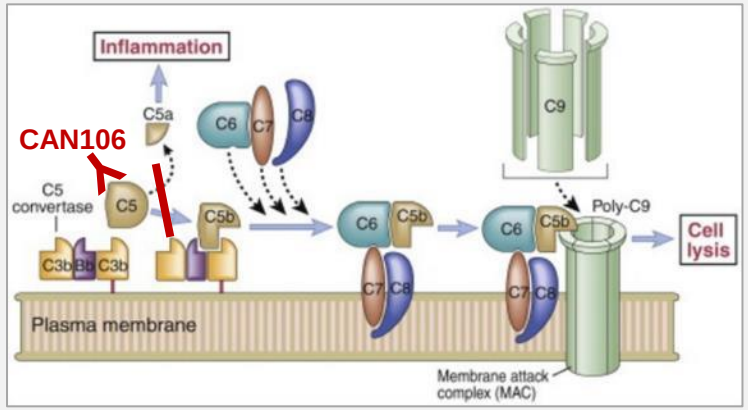
Obtained **global rights** to **develop, manufacture and commercialize** CAN106 through a strategic agreement with WuXi Biologics and Privus (Originator)

Favorable properties in **PD/PK study** with a prolonged duration of PD effect

Completed Phase 1 SAD study in healthy volunteers in Singapore and is currently in Phase 1b/2 study in patients with PNH in China (first PNH patient dosed in March 2022)

Safe and well-tolerated with mostly mild or moderate adverse events and no drug-related serious adverse events in Ph1 SAD study

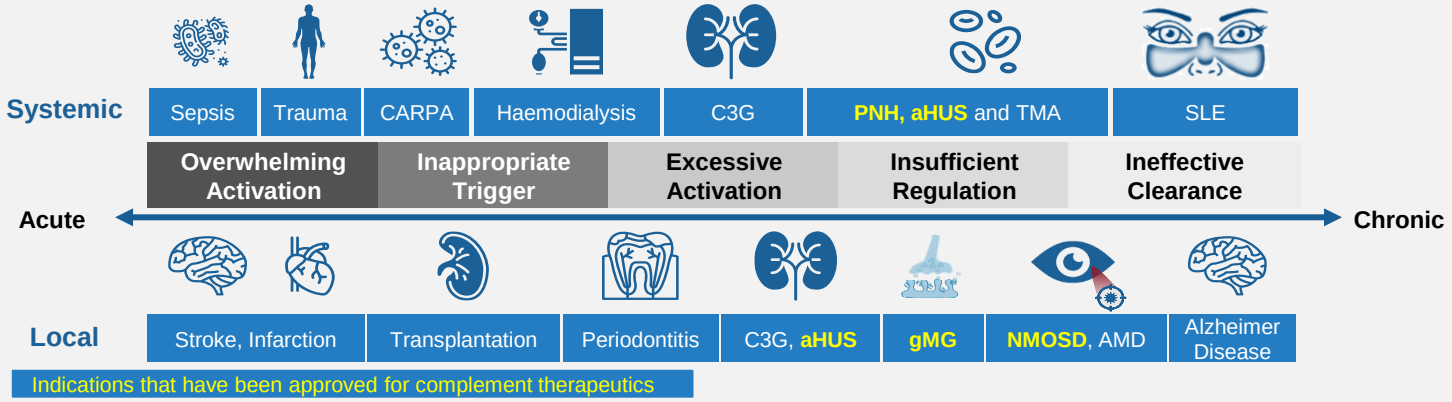
Mechanism of Action



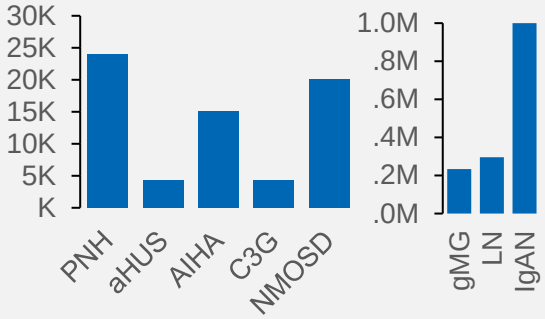
1. CAN106 binds to the α chain of C5, which prevents C5 from being cleaved into C5a and C5b by C5 convertase, thus preventing MAC formation and cell lysis
2. CAN106 preserves the generation of C3b, which is essential for the clearance of circulating immune complexes and the normal phagocytosis of bacterial and fungal pathogens

Potential "Pipeline in a Product". Initial development efforts focused on PNH treatment. Estimated global market revenue projections of >$9B in 2025[1]

Potential Indications for Complement Therapeutics



Estimated Addressable Patient Population in China[2]



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 cl, 2017; Zanella and Barcellini, 2014 & Berentsen and Sundic, 2015; Mahmoud et cl, 2016; CANbridge research

CAN106 – Phase 1 SAD Topline Results

Complete blockade of complement function encourages further studies in patients with PNH

SAD Topline Results

Safety

- CAN106 was safe and well-tolerated with no drug-related serious adverse events (SAEs)

Pharmacokinetics

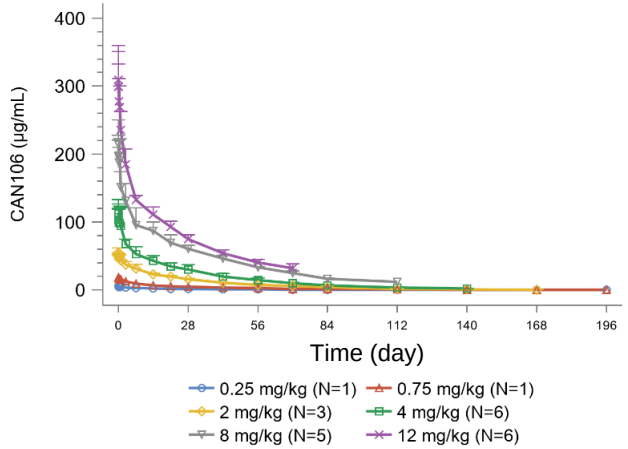
- CAN106 exposure (Cmax and AUC) was linear, dose-proportional, and had low inter-subject variability (<20% CV) with a half-life of 31 days

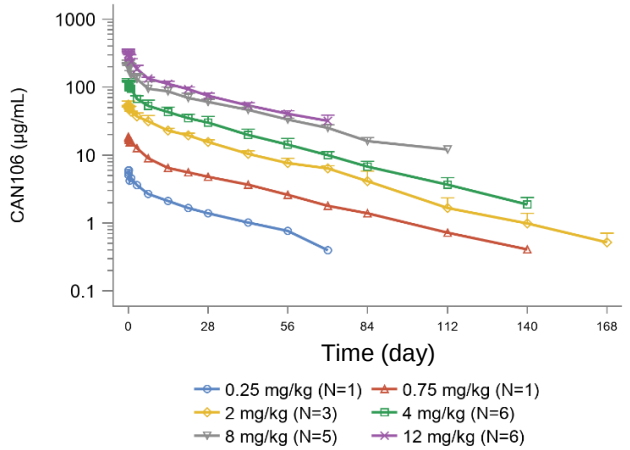
Pharmacodynamics

- CAN106 led to rapid and dose-dependent reductions in free C5 (target) and CH50 (serum hemolytic activity)
- Clinically relevant reduction in free C5 >99% and inhibition of CH50 >90% were achieved at the 8 and 12 mg/kg doses
- Complete complement blockade (CH50 >90% inhibition) was sustained for 2-4 weeks

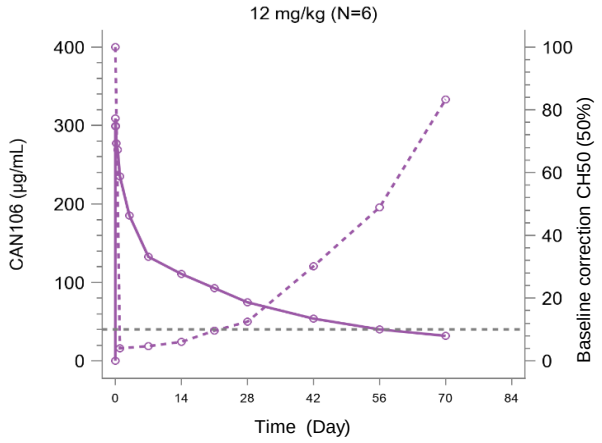
Study population 31 Healthy subjects
Primary endpoint Safety and tolerability
Secondary endpoint PK/PD (free C5 and CH50), Immunogenicity

Mean concentration-time curves of CAN106 by cohort



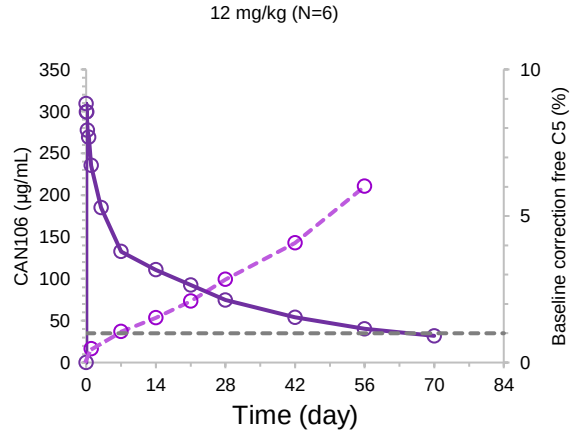


N (%) CH50 >90% reduction from baseline



Note: Baseline correction CH50 %) = post-dose CH50 / baseline CH50

Subjects with free C5 reduction >99% from baseline



Note: Baseline correction free C5 (%) = post-dose free C5 / baseline free C5

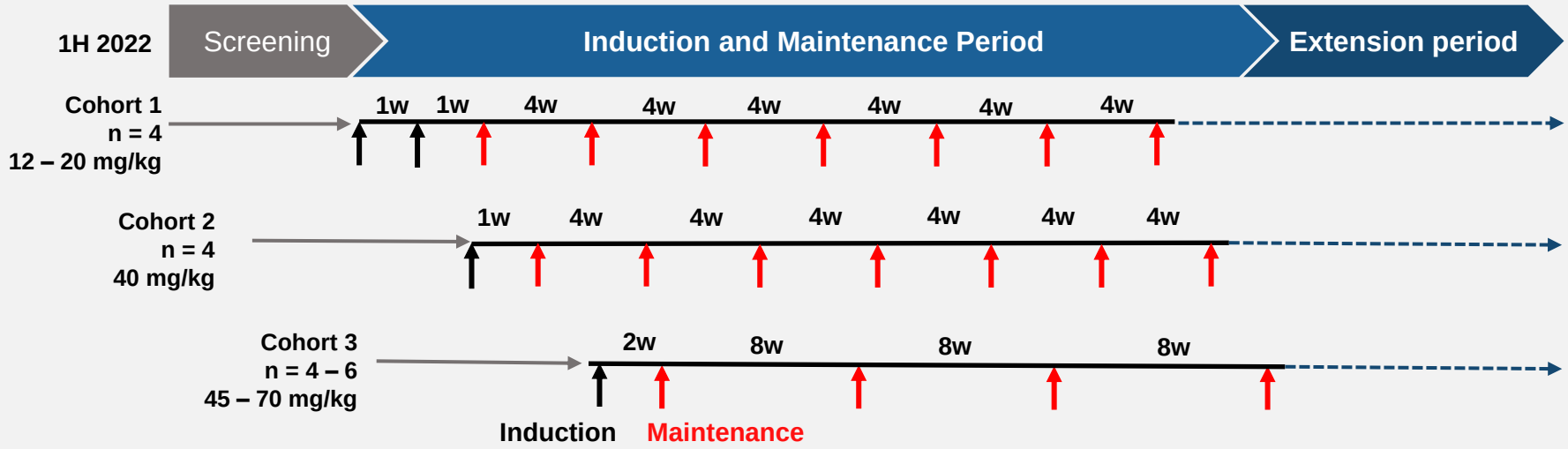
CAN106 Phase1b/2 Design for PNH in China

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

Study population
- Complement inhibitor treatment-naive patients with PNH

Primary endpoint
- Safety
- PK/PD

Region
- China



PNH
- Phase 1b FPD in March, Interim data analysis of Cohort 1&2 in 2H 2022
- Preparation of Phase 2 study will be initiated in 1H 2023 with patient enrollment in 2H 2023.
- Potential approval by China CDE in 2025

Other Indications
- Preparation of other programs will be initiated in 2H 2023
- Indications under considerations include: gMG, NMOSD, aHUS, etc.

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

Board will offer guidance on the CAN106 clinical 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

Neuromuscular Disorders

Robert Colvin, MD

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

Immunopathology of Kidney Disease and Organ Transplant Rejection

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

Rare Disease Drug Development

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 Women's Hospital
- Associate Professor of Medicine at Boston Univ Sch of Med

Organ Transplant, PNH, Thrombotic Microangiopathy

Richard Polisson, 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

Rare Disease Drug Development, Rheumatologic Diseases

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

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

Brian Weinshenker, MD

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

NMOSD and Other CNS Demyelinating Diseases

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

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

CAN008 Highlights

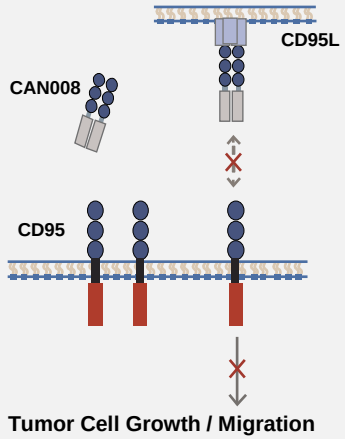
Obtained **exclusive rights** to **develop, manufacture and commercialize** CAN008 (APG101/asunercept) in Greater China from Apogenix

Fully human fusion protein that consists of extracellular domain of the CD95 receptor and the Fc domain of an IgG antibody

In a randomized, controlled Phase 2 study in recurrent GBM conducted by Apogenix, CAN008 showed statistically significant improvement in PFS and quality of life as well as a positive trend in OS

Currently in Phase 2/3 study in **newly diagnosed GBM** in China

Mechanism of Action



1. 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
2. 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
3. As the oligomerization of CD95 is blocked and signal transduction is inhibited, the growth and migration of tumor cells are suppressed

GBM Overview

A rare oncologic disease with **lower incidence** than other cancer types

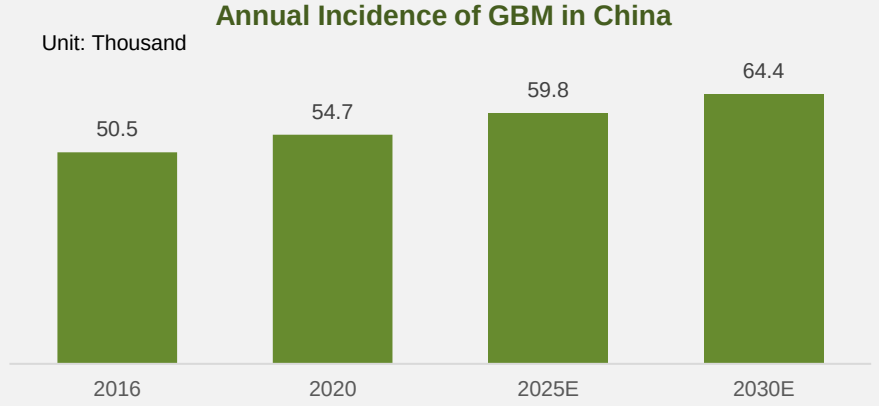
Median age of diagnosis is 64 years, and it is slightly more common (1.59 times) in men as compared to women

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[1], tumor treating field (TTF), bevacizumab (Avastin)

Epidemiology



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

CAN008 – Phase 1 Data and Phase 2 Design

Encouraging Phase 1 Data in newly diagnosed GBM[1]

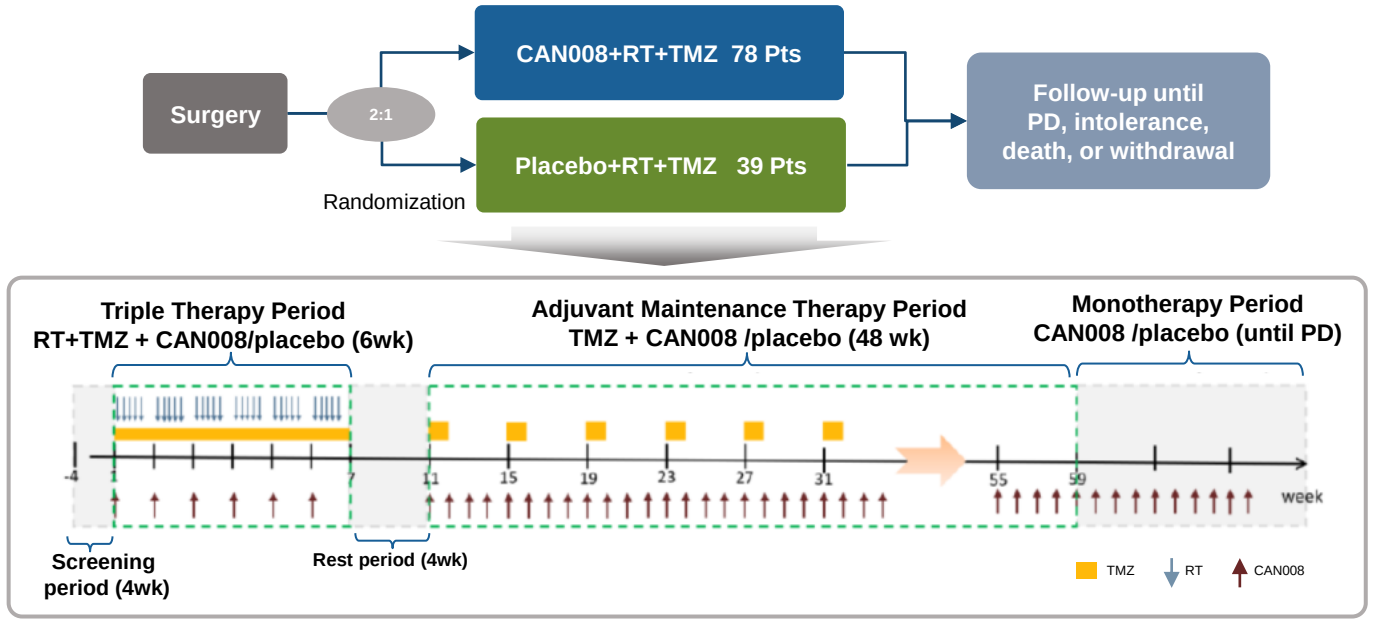
Safety

- **No specific safety issues** when CAN008 (200 or 400 mg) was combined with RT and TMZ.
- Two patients in Cohort 2 experienced serious adverse events (SAEs) **not related to CAN008.** Both patients recovered.
- **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.

Efficacy

PFS rates	Cohort 1 (200 mg; n=3)	Cohort 2 (400 mg; n=7)
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)

Phase 2/3 Multi-center, randomized, double-blind, placebo-controlled study



Study population

- Newly diagnosed GBM

Primary endpoint

- Progression-free survival (PFS)

Interim Readout

- Progression of 37 cases

To complete patient enrollment by 2H 2022

Source: 1, Wei K-C et al, Sci Rep 2021;11:24067

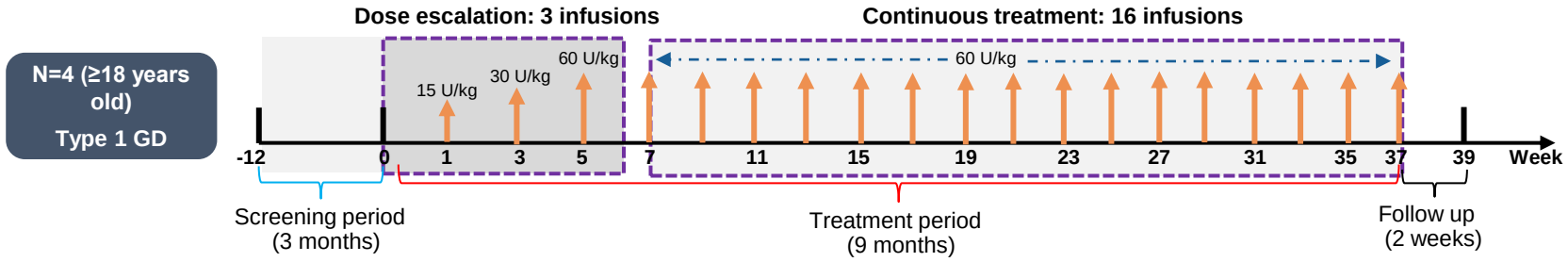
CAN103 First Patient Dosed in Phase 1/2 Trial

Multi-center Phase 1/2 trial consists of two parts and will recruit in total 40 adolescent and adult patients in China

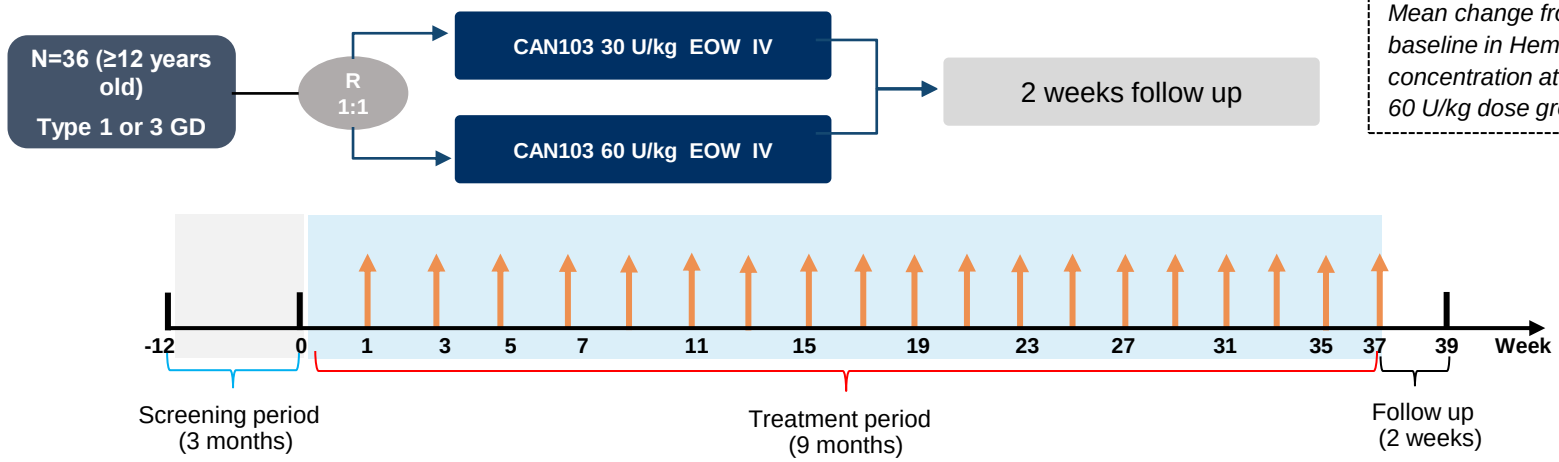
CAN103

- An **ERT** for **Gaucher Disease** (GD) developed in China
- The **first** rare disease asset acquired from WuXi Biologics, hold **global proprietary rights** to develop and commercialize
- One of the best known and prototypical rare diseases in China, approximately **3,000** patients in 2020
- **First patient dosed** in July 2022

Part A: Open-label, Within-subject, Dose escalation Phase 1 study



Part B: Randomized, Double-blind, Parallel-group, Dose comparison Phase 2 study



Primary Endpoint:
Mean change from baseline in Hemoglobin concentration at week 39 in 60 U/kg dose group

Abbreviation: GD, Gaucher Disease; EOW, every other week; IV, injection of vein; CAN103 is administrated intravenously every other week. One infusion; Note: all patients will enroll a long-term study after the Ph.1/2 trial

Three-Pronged Gene Therapy Research Strategy

In-house gene therapy research to build AAV platform for specific tissue targeting; accelerate development of cutting-edge gene therapy technology by partnering with industry innovators and working with academic experts

In-house Research

- Developing full-fledged gene therapy platform with AAV process development lab and pilot plants in Greater Boston area
- Targeting different tissue types, incl. central nervous system and muscle
- AAV process development lab expected to open in 2022

Close Partnership with LogicBio and Scriptr

- Using AAV sL65 capsid vector licensed in from LogicBio to develop two gene therapy products for the treatment of Fabry disease and Pompe disease and technology from Scriptr to develop treatment for DMD
- Options to develop two additional indications using the same vector and a clinical-stage gene editing program for the treatment of methylmalonic acidemia from LogicBio

LogicBio THERAPEUTICS

Scriptr™ GENETIC SOLUTIONS FOR GOOD

Strategic Collaboration with Leading Research Institutions

- Initiated research programs with the Horae Gene Therapy Center at the UMass and UW to develop gene therapy solutions for neuromuscular disorders
- Have the exclusive option to license-in the UMass asset for development
- Potentially among the first China-based companies to commence global-level collaboration in AAV gene therapy

University of Massachusetts Medical School
UMASS

UW Medicine
UW SCHOOL OF MEDICINE

CANbridge Innovative AAV Platform

Features

- Liver de-targeted AAV to avoid peripheral sinkers
- No impact on productivity
- One AAV "fits all"
- Reprogrammable for single or multi-tissue delivery
- NAb evasion – accessible to all patients
- Simplify manufacturing process development

Fixed AAV capsid allow us to:

- Use the best AAV manufacturing platform
- Save cost on development
- Use single manufacturing process
- Same analytical assays
- Reduce COGs = improved affordability and patient access
- Increase speed to market

LogicBio Pre-Clinical Data[1]

- **Highly efficient** functional transduction of human hepatocytes.
- **Improved** manufacturability
- **More resistance** to **pre-existing neutralizing antibodies** in human serum samples

Collaboration with Gene Therapy Experts

Dr. Guangping Gao

- Strategic advisory board member for gene therapy collaboration with UMass
- Has authored **250+** research papers and holds **131** patents and **221** pending applications
- Co-founder of **Voyager Therapeutics** and **Aspa Therapeutics**

Dr. Jeffrey Chamberlain

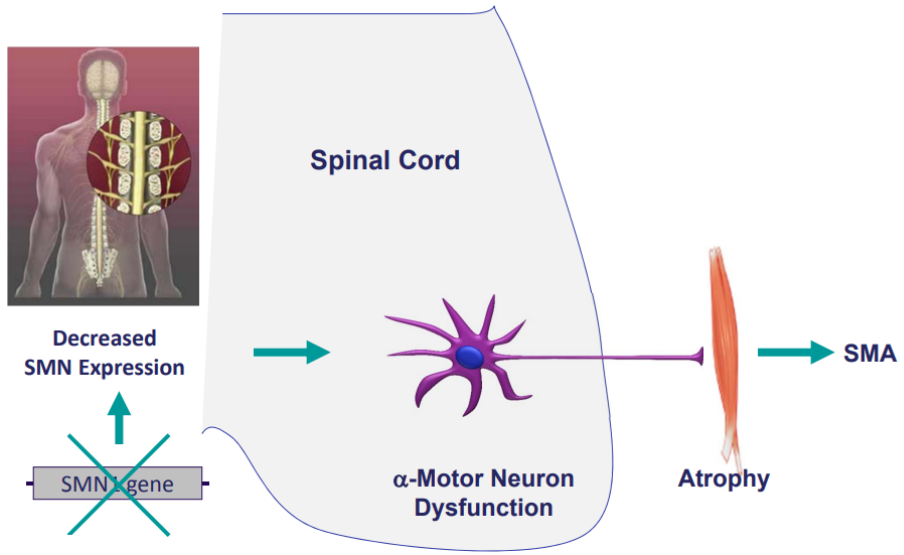
- The McCow Endowed Chair in Muscular Dystrophy, UW, School of Medicine; Council Member, American Association for the Advancement of Science; VP of ASGCT
- Has authored **110+** research papers (GT and DMD)
- Scientific advisory board of **Solid Biosciences**

Notes: 1, Presented at the American Society of Gene & Cell Therapy Conference in May 2020

UMass Collaboration: Gene Therapy for Spinal Muscular Atrophy

The first and only gene therapy approved for SMA expected to hit peak global sales of $3 billion[1]

Pathophysiology Illustration[2]



- SMA is characterized by dysfunction of α-motor neurons
- 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[2]

- Autosomal recessive genetic inheritance
- 1 in 50 people are carriers
- 1 in 6,000 to 1in 10,000 children born with SMA
- One of the most common rare diseases
- Affects all racial and ethnic groups

Approved Targeted Therapies

- Gene therapy (e.g., Novartis's Zolgensma®)
- Correct splicing (e.g., Biogen's Spinraza® and Roche's Evrysdi®)

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

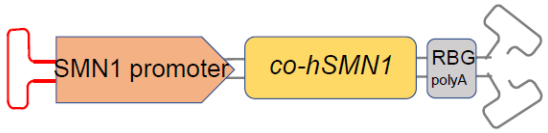
Source: 1, Cowen equity research. 2, Adapted from SMA foundation

CANbridge-UMass SMA Program 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

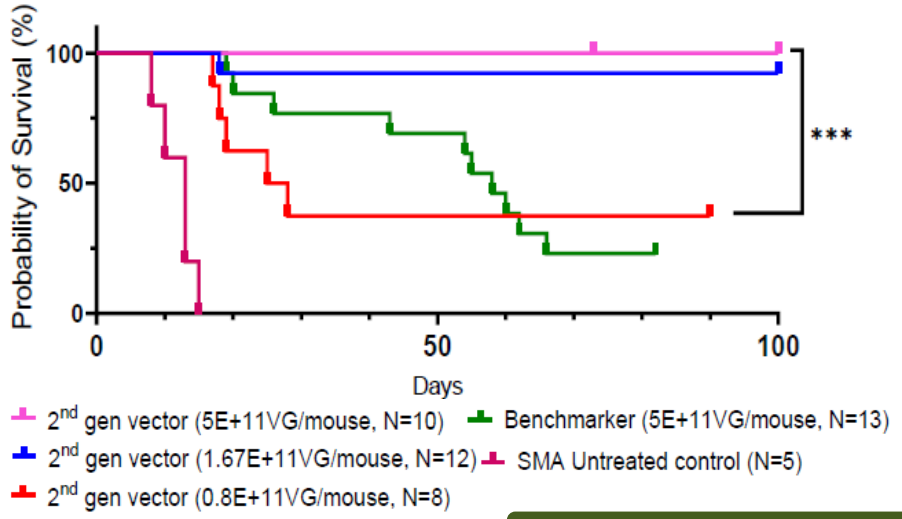
UMass Chan MEDICAL SCHOOL
Horae (红瑞) Gene Therapy Center

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

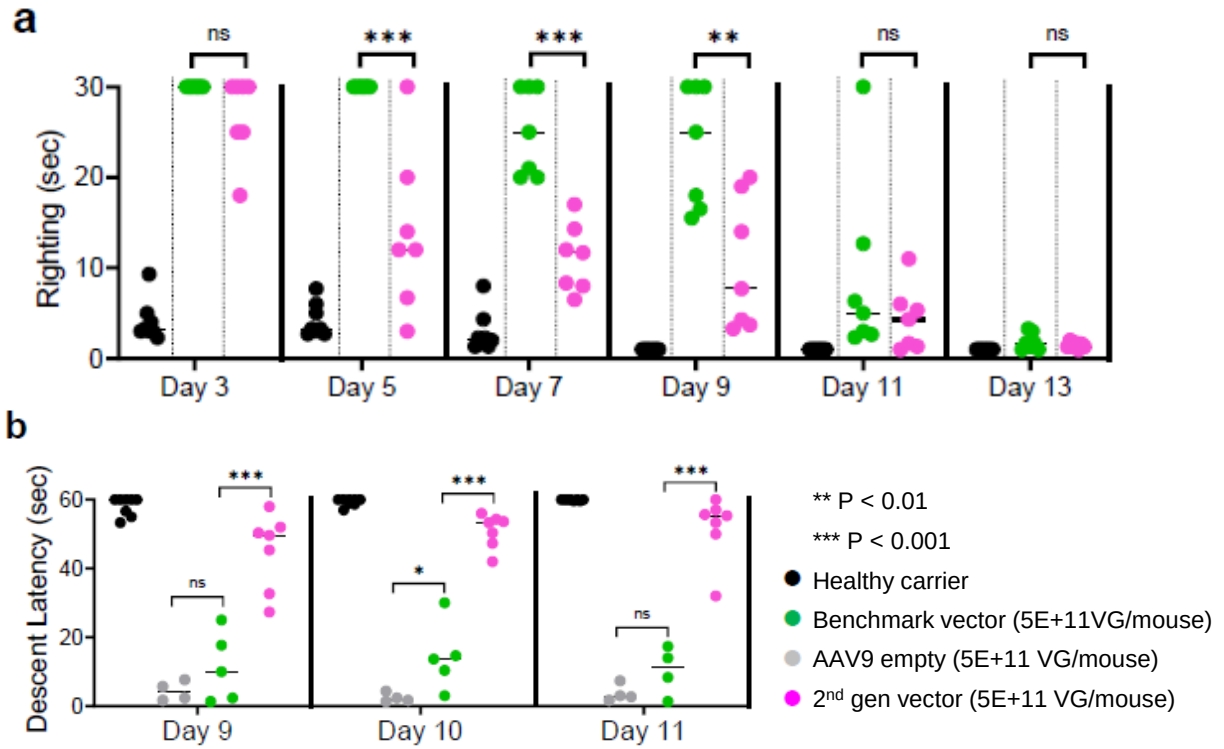


pAAVsc-*SMNp-co-hSMN1* (2nd gen vector)

Survival curve of vector-treated mice



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

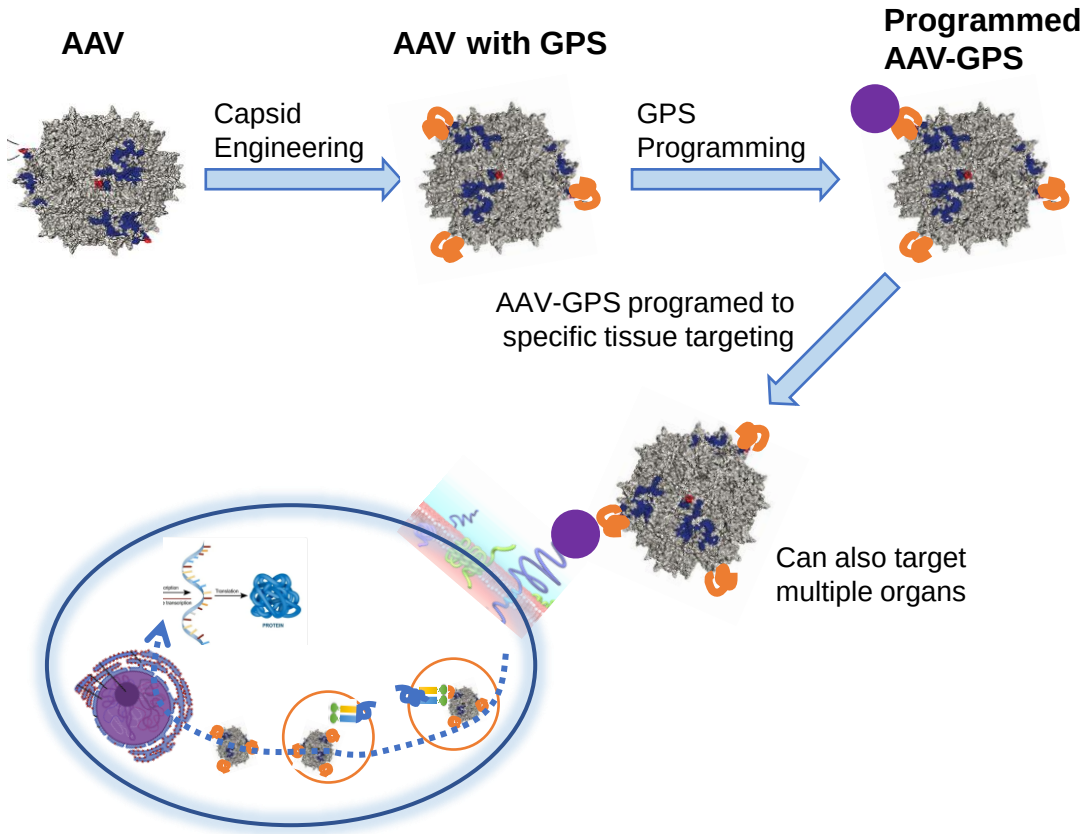


In vivo data 2nd gen vector demonstrate advantages in extension of life span, elimination of liver toxicity, and improved restoration of muscle function

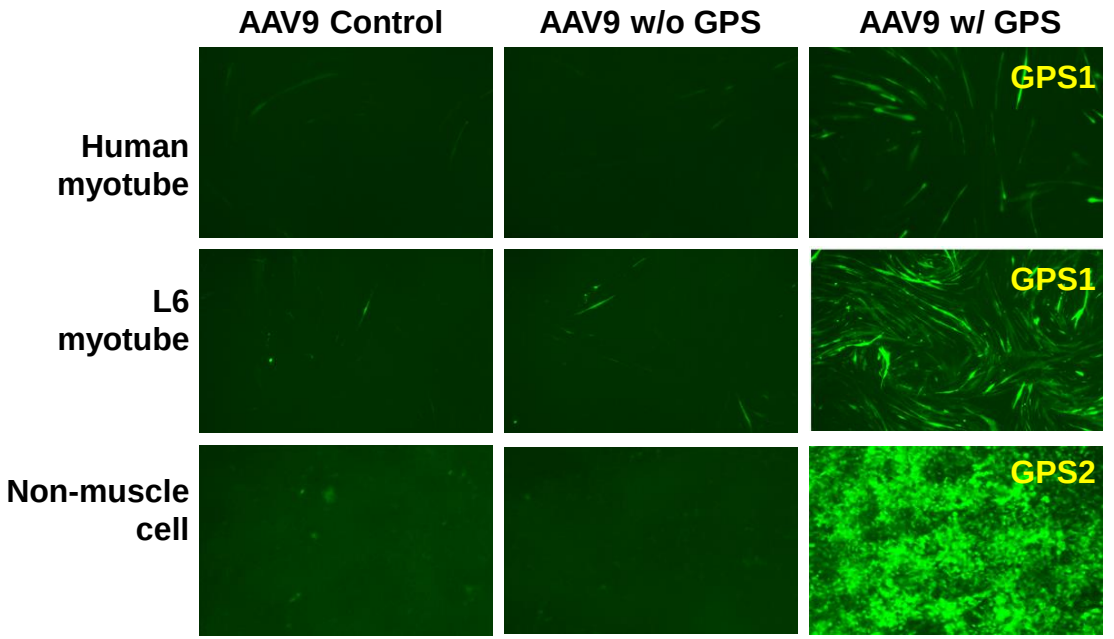
Source: https://annualmeeting.asgct.org/abstracts/abstract-details?abstractId=6140

CANbridge Innovative AAV Platform: AAV-GPS

CANbridge AAV-GPS Tissue Specific Delivery Platform



PoC of AAV-GPS *in vitro* in Myotubes and Non-Muscle Cells



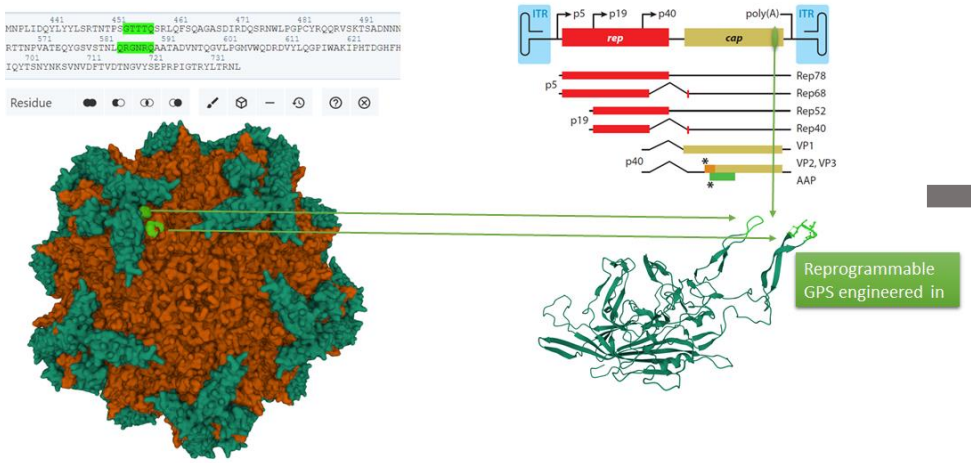
Additional Data

- AAV-GPS demonstrated superior transduction to AAV9, with similar transduction to MyoAAV*
- IVIG inhibited transduction of myotubes by AAV9 and MyoAAV, but not by AAV-GPS

Note: Tabebordbar M et al, Cell 2021

Comparison of CANbridge AAV-GPS with Other Novel Capsid Approaches

	Natural Cap Discovery	Rational Design	Directed Evolution	In-Silico Design
Pros	• 100s identified • Nature made from evolution	• Based on understanding of receptor biology • More predictable species translatability • May alter NAb	• High throughput • Inclusive of all possible combinations nature can't do	• As for Directed evolution • Powerful AI learning capsid biology to guide rational library design
Cons	• Serendipitous • Broad tissue tropism • NAb/CTLs • Unknown species translatability	• Lack of full understanding of receptor biology • Hard to engineer into AAV	• Time consuming on panning Unknown species translatability • Costly validation on transability and manufacturability • **Different tissues may need different AAVs**	• As for Directed evolution • **Different tissues may need different AAVs**



CANbridge AAV-GPS

- Small GPS size to avoid impact on CMC developability
- Liver de-targeted AAV to avoid peripheral sinkers
- One AAV fits all, only need to replace the “GOI”
- Simplify manufacturing process development with one AAV
- Programmable GPS for a specific tissue or tissues
  - Single or multi-tissue delivery capability
- Bypass NAb → increases the number of patients eligible to AAV gene therapy
- Two targets on the same tissue can be used by two different GPS to further avoid NAbs for repeated dosing

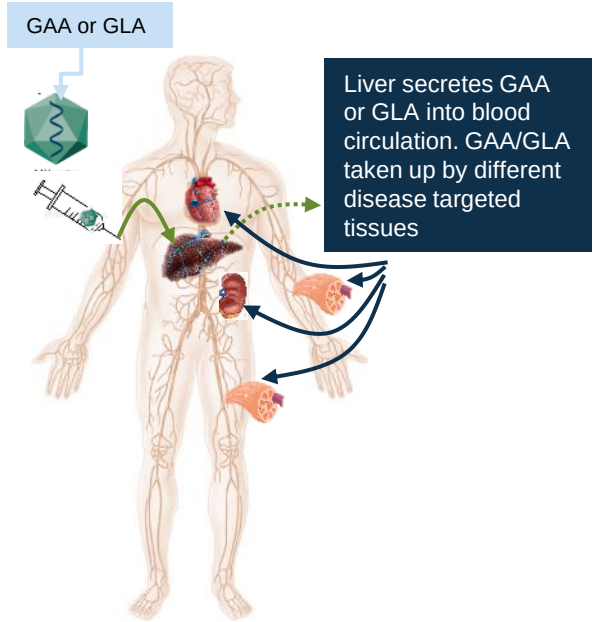
Developing Gene Therapies to Treat LSDs

Gene therapy holds the promise to transform treatments for LSDs such as Fabry disease / Pompe disease from chronic to curative

Application to Lysosomal Storage Diseases (LSDs)

LSDs are a group of over **70 diseases** that are characterized by lysosomal dysfunction, most of which are inherited as autosomal recessive traits, including Gaucher disease, Fabry disease and Pompe disease

Clinical trials are in progress on possible treatments for some of these diseases, but there is currently **no approved treatment** for many LSDs



	CAN201 - Fabry disease (FD)	CAN202 - Pompe disease (PD)
Introduction	One of the most common LSDs, usually starts in childhood	One of the most common LSDs, onset of symptoms from childhood to adulthood
Prevalence *(in Thousand)*	2016: 354.0 (China) / 1,359.6 (Rest of the World) 2020: 360.3 / 1,428.3 2025E: 364.6 / 1,512.1 2030E: 368.3 / 1,591.1	2016: 37.4 (China) / 125.8 (Rest of the World) 2020: 38.2 / 132.2 2025E: 38.7 / 140.1 2030E: 39.3 / 147.4
Treatment Approaches	Symptomatic Treatment; ERT; Substrate reduction therapy; Chaperone therapy	Symptomatic treatment; ERT

Gene therapy is considered an innovative and promising treatment and is currently at clinical stages

03

Outlook

Upcoming Key Milestones

We expect in 2H 2022 and 2023:

*As of 30 June, 2022, we have cash balance of **$604.6M RMB****

- **CAN106** – Phase 1b/2 interim data analysis of Cohort 1&2 in Q422/Q1 23
- To complete **CAN008** Phase 2 patient enrollment by EOY 2022
- Multiple pre-clinical data on gene therapy programs

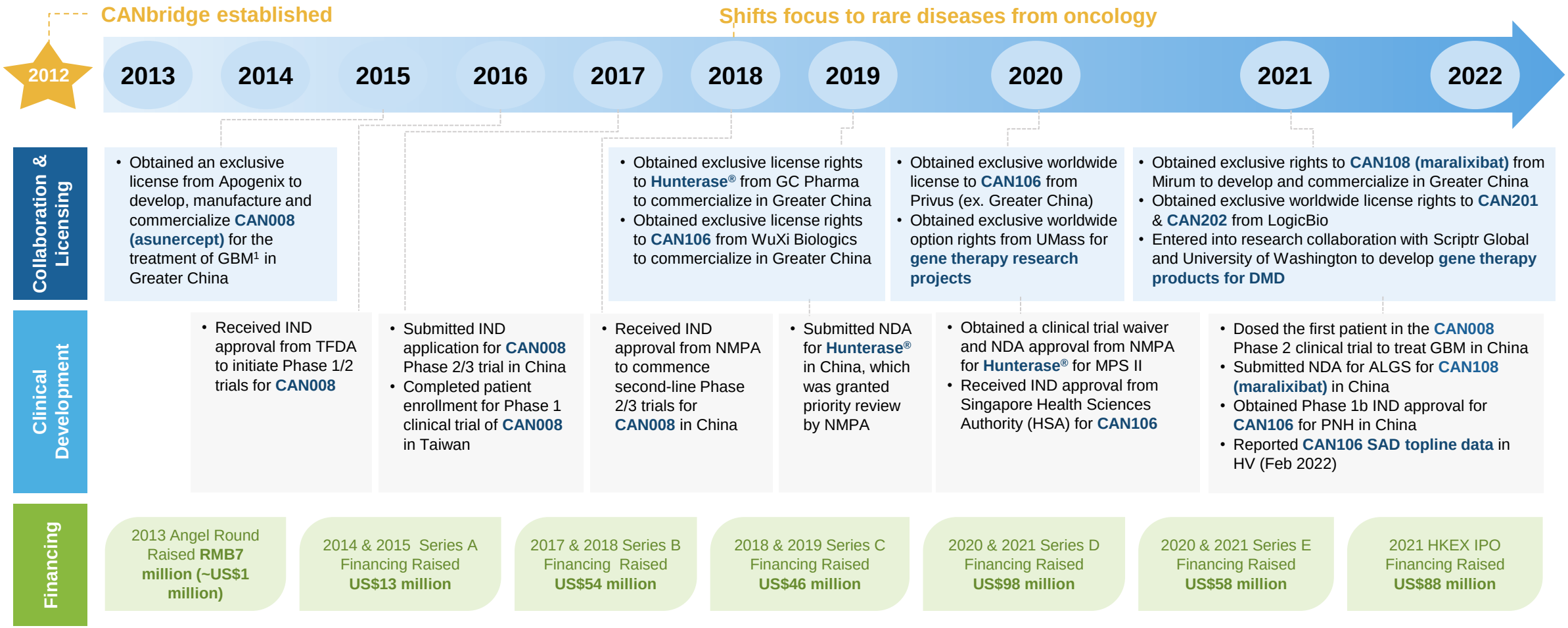
- **CAN108** – Potential approval for ALGS
- **CAN008** –Phase 2 interim readout in 2023
- Multiple pre-clinical data on gene therapy programs

1 USD = 6.70 CNY, as of 30 June, 2022

04

Appendix

Track Record of Sourcing and Developing Innovative and Validated Therapies



Abbreviation: GBM, glioblastoma multiforme