CANbridge Pharmaceuticals

FY2021 Annual Results Presentation

March 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

CANbridge at a Glance

We are a leading developer of rare disease treatments for the Chinese and global markets, committed to the research, development and commercialization of innovative therapies with massive market potential

A Pioneer in the China Rare Disease Market

- Establish the **rare disease ecosystem** in China by working closely with **key stakeholders**
- Access to **a large treatment-naive patient pool**
- Have established **a strong infrastructure**

Regulators | KOLs | Doctors | Patients

Center of Excellence and Reimbursement | Insurance Institutions

Comprehensive Pipeline with Significant Revenue Potential

- Target rare disease and rare oncology indications
- Select candidates **with validated mechanisms of action**
  - Cross multiple modalities: **biologics**, **small molecule drugs, gene therapies**
    - **13 drug assets** for the treatment of **rare diseases** and **GBM** in China and global market, as well as **genetic diseases** based on **next-gen platform**

CANbridge

Experienced management team with deep industry expertise and strong track record led by a visionary founder

Extensive Global Collaborations

- **Industry**: Successful **in-licensing of innovative** and **validated therapies from global innovators** followed by rapid advancement to commercialization
- **Patient Advocacy Groups**: CEO is the **Deputy Director General of China's Alliance for Rare Disease (CHARD)**
- **Research/Academic Institutions:** Seek **"best of" technologies** to advance in-house development

License-in Partners: apogenix, mirum, GC Pharma

Research Co-developers: Privus, LogicBio, Scriptr, WuXi Biologics, University of Massachusetts Medical School, UW Medicine

Fully Integrated Platform

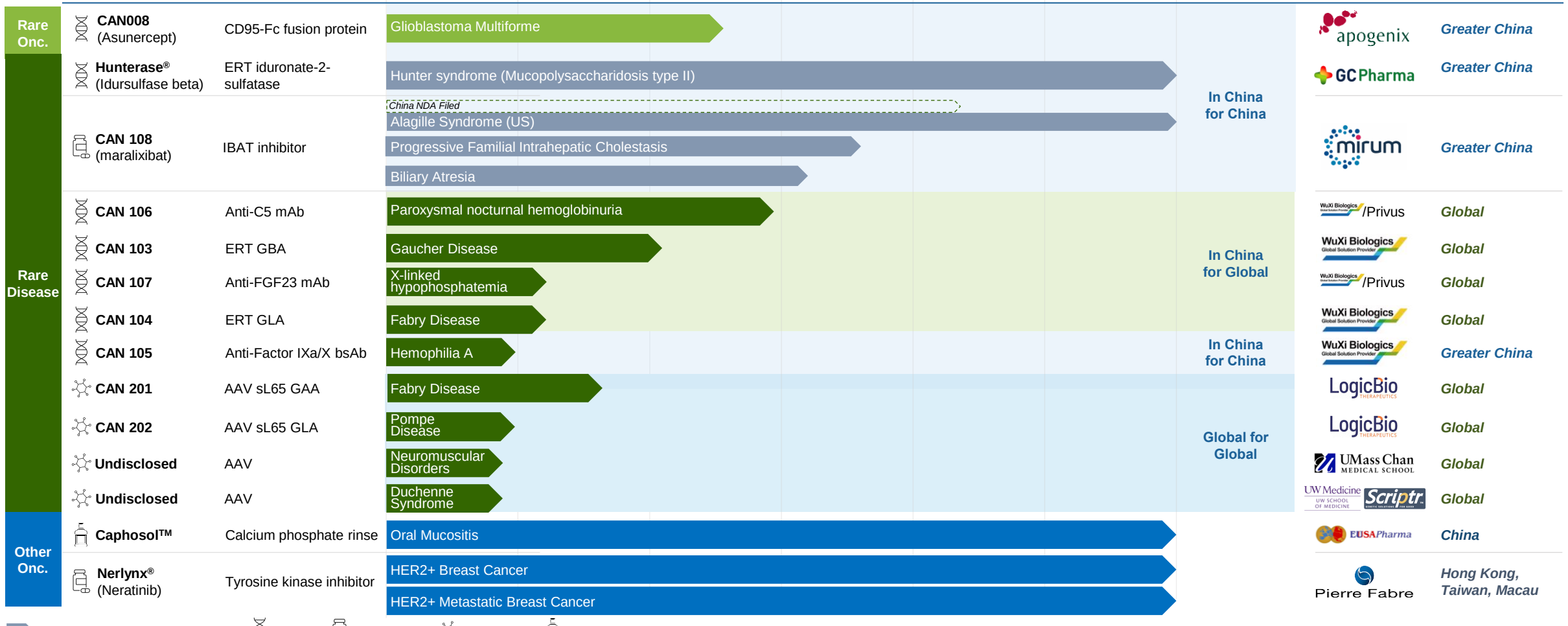
- Cover **the entire spectrum of drug development**

Early discovery/ Preclinical research | **Clinical development**

Manufacturing | **Commercialization**

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	Neuromuscular disease			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
- Open 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

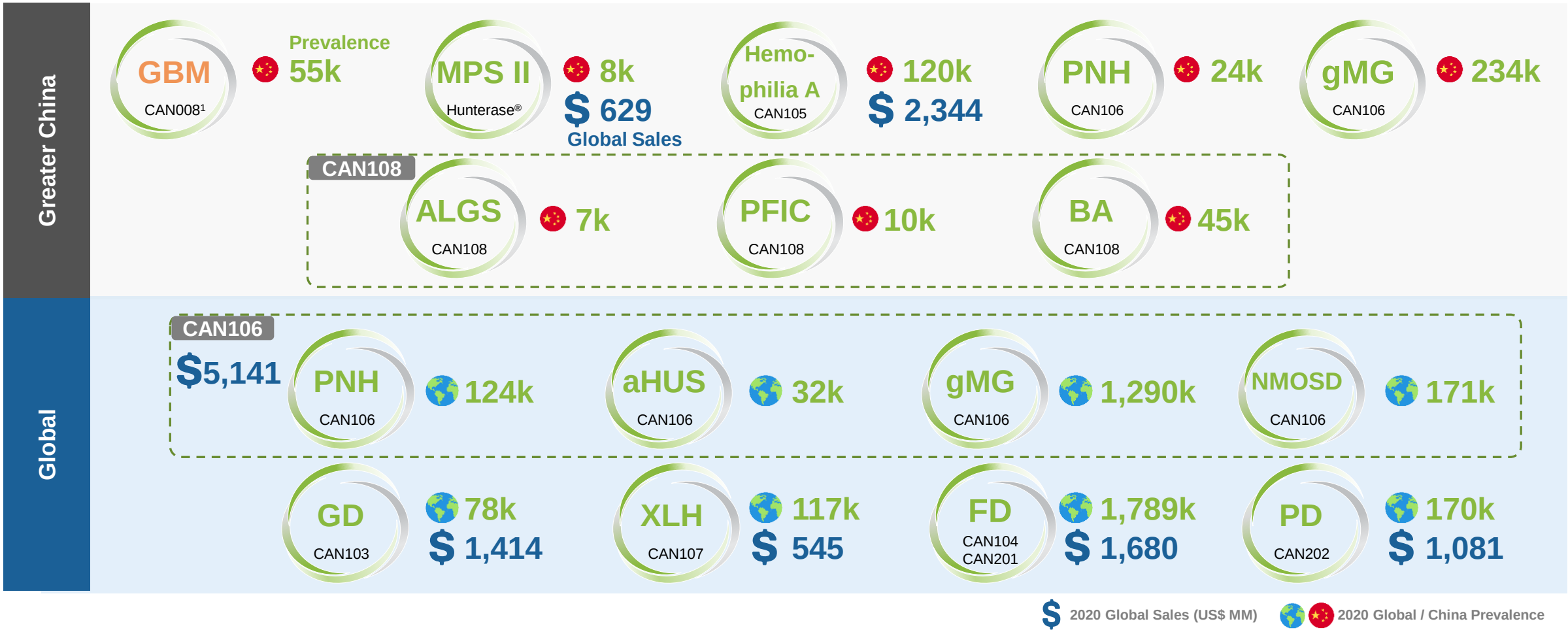
- Using tissue specific cell surface receptors for targeting
- AAV platform enables future development in multiple CNS/muscle-related diseases
- Patent filing in process

Abbreviations: FTE, full-time equivalents

CANbridge

Pipeline Targets Diseases with Significant Revenue Potential

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



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 Analysis, NCBI research, Endocrine Journal research, World Federation of Hemophilia research Notes: 1. CAN008 currently has no commercialized comparable product

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

Dr. Yunxiang Zhu

Vice President, Head of Global Research

- **~20 years** of R&D leadership experience in the biotechnology industry
- Former Senior Vice President at Shenogen Pharma Group
- Former senior director at Sanofi Genzyme, **led the invention of the second-generation enzyme replacement therapy**

Marcelo Cheresky

Chief Business 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

Jeff Lau

Vice President of Finance and Controller

Rebecca Zhang

Vice President of Regulatory Affairs

Lily Liu

Head of Market Access

Stella Mao

Director, Public Affairs

Qionghui Qiu

Director of Clinical Operation

Wei Zhang

Director & China Head of CMC Department

Chris Chen

Senior Director of Human Resources

Jenny Tao

Director, Quality Assurance

Business Highlights in 2021

Corporate and Business Development

- FY2021 net revenue **RMB 31.2M**, mainly attributable to sales from Hunterase in mainland China and Nerlynx in HK/TW
- Strategic Collaboration with **LogicBio Therapeutics** and Licenses to Gene Delivery and Editing Platforms
- Obtained an exclusive license from **Mirum** to develop, manufacture and commercialize CAN108 in Greater China for ALGS, PFIC and BA
- Entered into a research collaboration and license agreement with **Scriptr Global** and **University of Washington**
- Established a **US-based discovery lab** in Natick
- Listed on Main Board of **Hong Kong Stock Exchange**

CAN008

- IND application amended to allow CAN008 to be studied as a **first-line** Phase II trial Initiated a **Phase II trial** in China in April 2021 and dosed the first patient in October

CAN108

- **NDA for ALGS** accepted and granted priority review status by NMPA in January 2022

CAN106

- Completed a Phase 1 study in Singapore
- Obtained the IND approval from the NMPA for **PNH** in July 2021, initiated a **Phase 1b/2a study** in China in December 2021
- Reported positive topline Phase 1 data from Singapore trial in February 2022

CAN103

- Obtained the **IND approval** for CAN103 from the NMPA in October
- Currently in preparation to begin a Phase 1 trial in adult and adolescent Gaucher disease patients

Gene Therapies

- Initiated two programs: **CAN201** for treatment of Fabry disease and **CAN202** for the treatment of Pompe disease

02

Pipeline Update

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

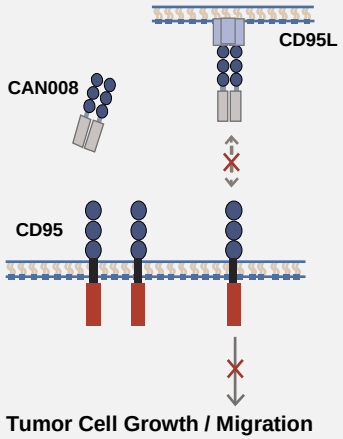
Obtained **exclusive rights** to **develop, manufacture and commercialize** CAN008 (APG101/asunercept) in Greater China from Apogenix

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

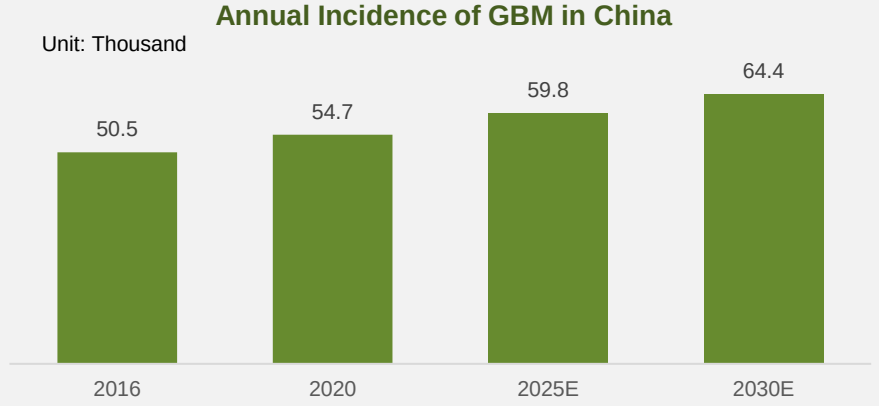
Median age of diagnosis is 64 years, and it is slightly more common (1.59 times) in men as compared to women

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]

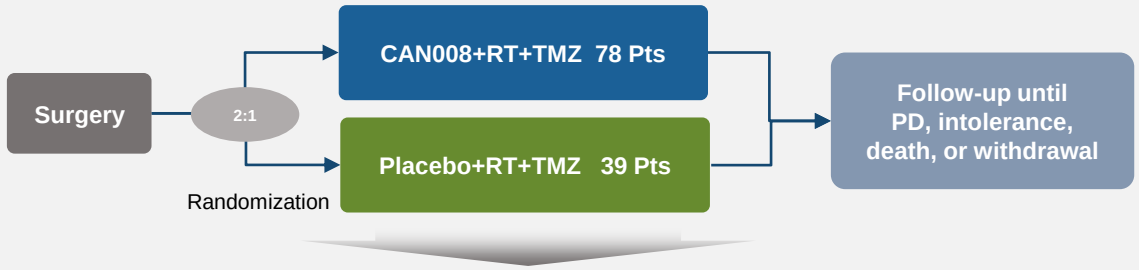
Safety

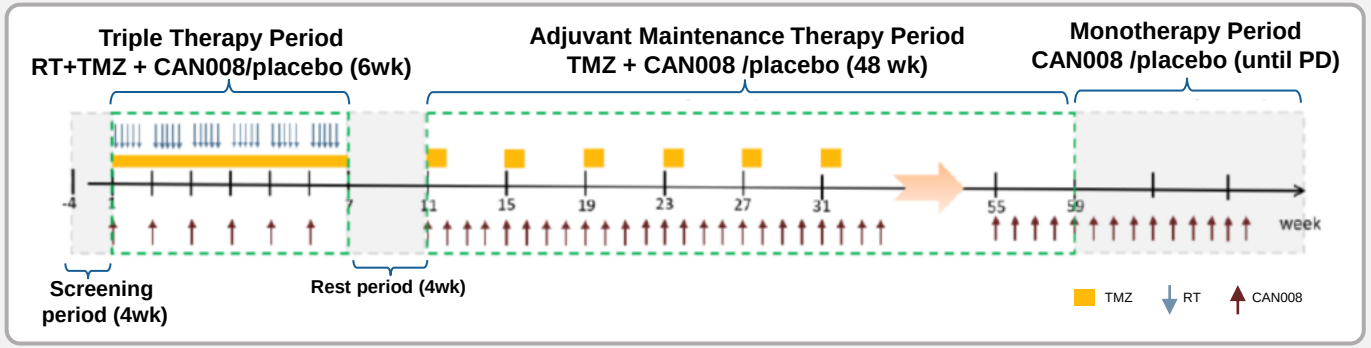
- **No specific safety issues** when CAN008 (200 or 400 mg) was combined with RT and TMZ.
- Two patients in Cohort 2 experienced serious adverse events (SAEs) **not related to CAN008.** Both patients recovered.
- **No subjects discontinued** due to treatment-emergent adverse events.
- No patients experienced 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 Multi-center, randomized, double-blind, placebo-controlled study





Study population
- Newly diagnosed GBM

Primary endpoint
- Progression-free survival (PFS)

Secondary endpoints
- Overall survival (OS)
- 6-month rate of progression-free survival (PFS6)
- Objective response rate (ORR)
- Cognitive function determined by MMSE
- Quality of life (QoL)

Interim Readout
- Progression of 37 cases

*CAN008 is administered 400mg IV once weekly until disease progression or unacceptable toxicity.
GBM: Glioblastoma multiforme RT: Radiotherapy
TMZ: Temozolomide PD: Progressive disease

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

Hunterase® – The Only ERT Approved for MPSII Launched in China

Large untapped MPSII market in China. Patient identification and treatment reimbursement key to unlocking full commercial potential

Hunterase® (海芮思)

- ❑ Launched in May 2021
- ❑ Indicated for patients with Hunter syndrome (MPS II)
- ❑ The first ERT for treating MPSII approved in China

Overview of MPS II

- Hunterase® is the **first and only approved treatment** of MPS II in China
- 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 Marketing Strategies

China Treatment Consensus	**Early use of ERT** upon diagnosis is recommended as it improves patient prognosis
HCP Education	**96%** of surveyed physicians reported aided or spontaneous recall of Hunterase in China*
Patient Identification/ Diagnosis Projects	**195** newly identified MPSII patients out of **~8,000** potential patients in China
Reimbursement Campaign	**3** provinces & **20** cities Hunterase covered by commercial insurance

Source: * based on a market research study conducted by CANbridge in Jul 2021

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

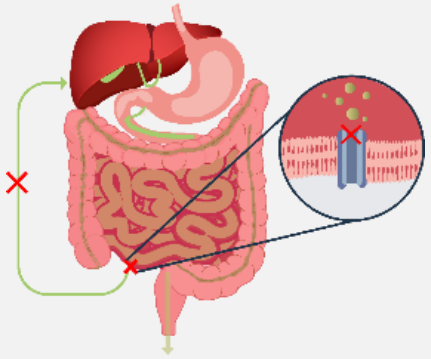
Obtained an **exclusive license** to develop, manufacture and commercialize Livmarli (maralixibat) in Greater China from Mirum

Approved by the **FDA** for the and **symptoms** in targeted settings and provide an **alternative treatment** totreatment of **ALGS** in the U.S. in September 2021. Priority review granted by NMPA. 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

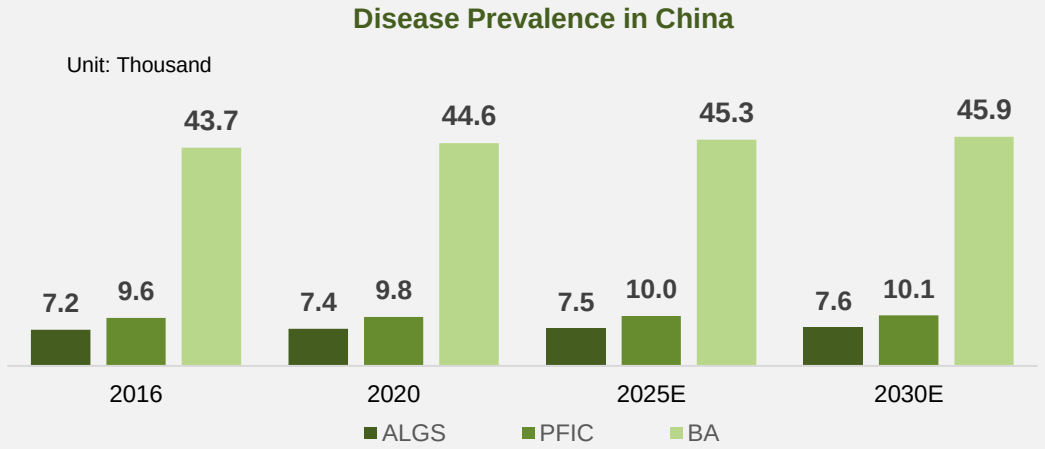
Disease Overview

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

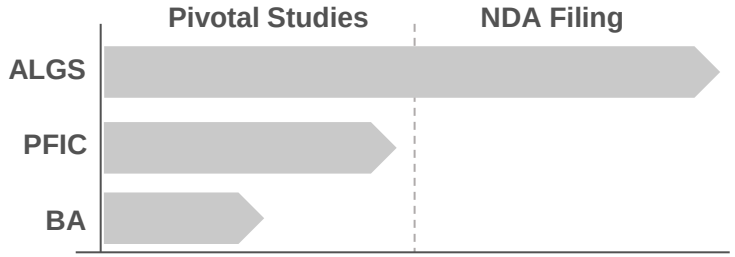
CAN108 – Clinical Development Plan

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

Collaboration with Mirum for the development of CAN108

*CANbridge obtained **exclusive license** from Mirum to develop, manufacture and commercialize maralixibat in Greater China. in April 2021. **US FDA** approved Livmarli (maralixibat) for ALGS in September 2021.*

CAN108 Development Status



ALGS: China NDA potential approval in Q1 2023; TW/HK potential approval in 2H 2023. **Special early access program** has been initiated in Boao (Hainan province)

PFIC: File NDA after Mirum filing, potentially in early 2023

BA: Support patient recruitment and Chinese clinical site management as part of Mirum's global Phase 2 clinical trial, initiated in 1H2021

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

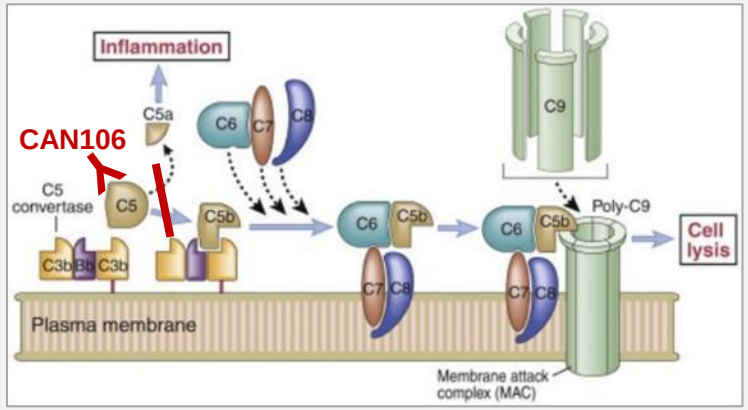
Obtained **global rights** to **develop, manufacture and commercialize** CAN106 through a strategic agreement with WuXi Biologics and Privus (Originator)

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

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

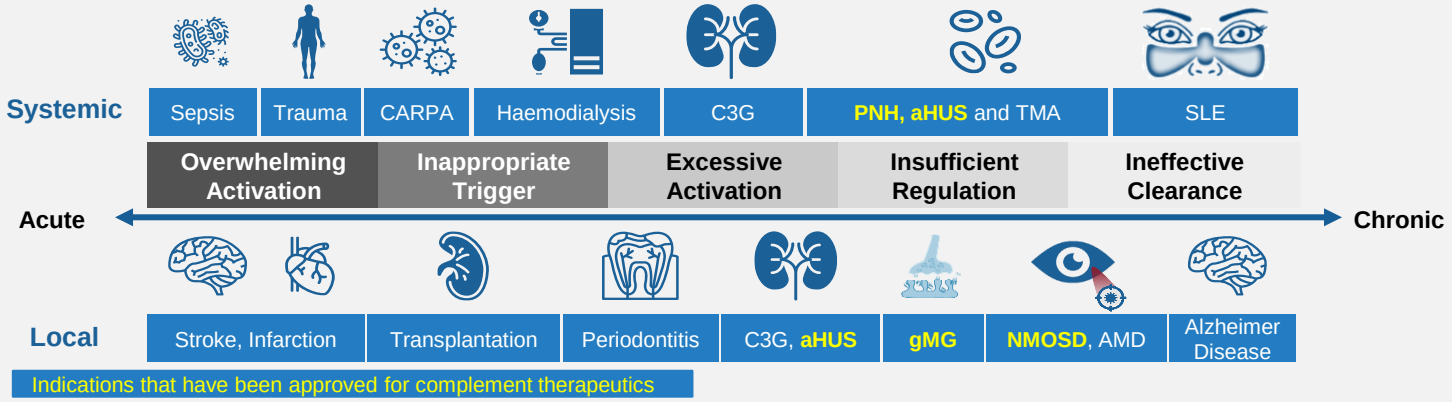
Mechanism of Action



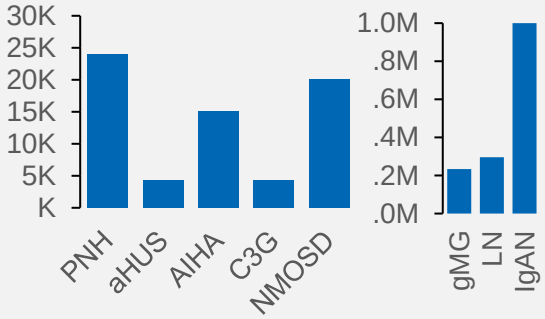
1. CAN106 binds to the α chain of C5, which prevents C5 from being cleaved into C5a and C5b by C5 convertase, thus preventing MAC formation and cell lysis
2. CAN106 preserves the generation of C3b, which is essential for the clearance of circulating immune complexes and the normal phagocytosis of bacterial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

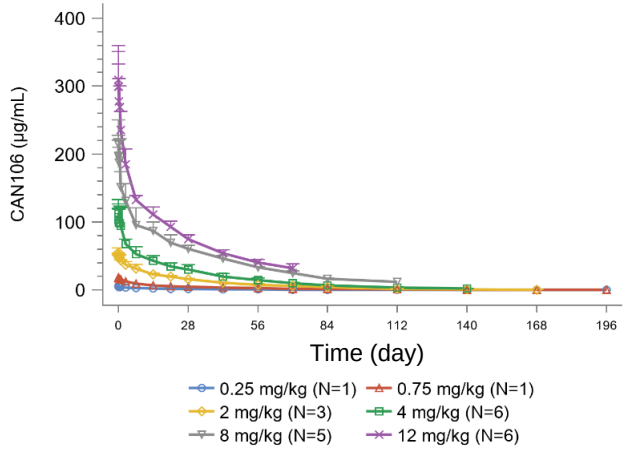
- CAN106 exposure (Cmax and AUC) was linear, dose-proportional, and had low inter-subject variability (<20% CV) with a half-life of 32 days

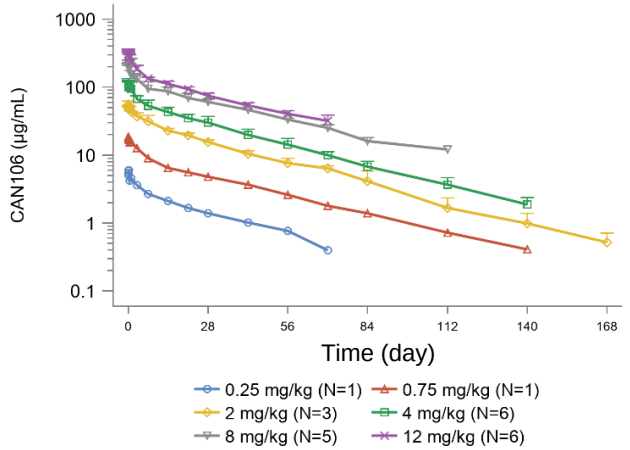
Pharmacodynamics

- 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

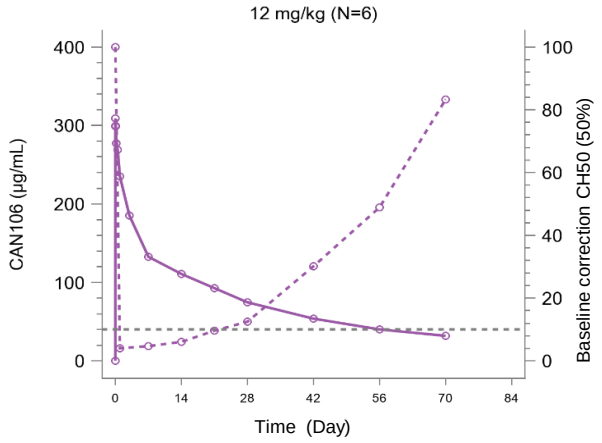
Study population 31 Healthy subjects
Primary endpoint Safety and tolerability
Secondary endpoint PK/PD (free C5 and CH50), Immunogenicity

Mean concentration-time curves of CAN106 by cohort



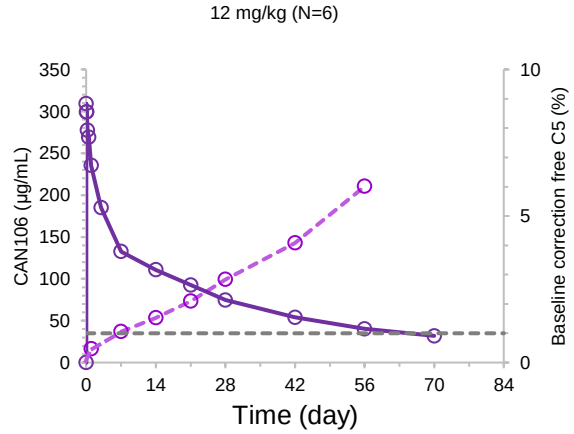


N (%) CH50 >90% reduction from baseline



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

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

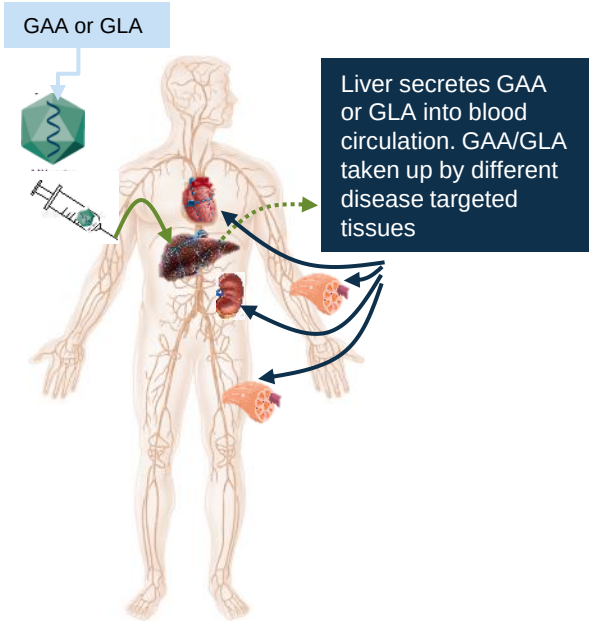
Developing Gene Therapies to Treat LSDs

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	A rare genetic condition and the first identified LSD
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 / 125.8 2020: 38.2 / 132.2 2025E: 38.7 / 140.1 2030E: 39.3 / 147.4
Treatment Methods	Symptomatic therapy; ERT; Substrate reduction therapy; Chaperone therapy	Symptomatic treatment; ERT

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

CAN103, CAN107, CAN105 and CAN104 – Preclinical Candidates

Global Prevalence ~78k

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
- **IND approved by NMPA** in 3Q 2021
- Plan to conduct a Phase 1/2 trial in adult and adolescent GD patients

Global Prevalence ~141k

CAN107

- A **recombinant humanized anti-FGF23 mAb** for **X-linked hypophosphatemia** (XLH) being developed in China. At pre-IND CMC stage
- XLH is an **inherited disease** of phosphate metabolism in which mutations inactivating the phosphate regulating endopeptidase homolog, X-Linked (PHEX) gene lead to inactivation of the PHEX protein
- Disease prevalence is 1 in **20,000 (est)**

China Prevalence ~120k

CAN105

- Under development for the treatment of **hemophilia A** with **massive market potential**
- A recombinant, humanized, bispecific antibody that bridges activated factor IX and factor X to restore the function of the missing activated factor VIII
- **Over 120,000** hemophilia A patients in China in 2020 (est)

Global Prevalence ~1,789k

CAN104

- An **ERT** for the treatment of **Fabry disease**
- Exogenous delivery of intravenous recombinant human α-GAL can replace GAL activity in patients with decreased or absent enzyme activity, reducing GL-3 storage and slowing the progression of renal disease
- There are **five approved ERTs for Fabry disease globally**

Source: Frost & Sullivan Analysis, Ultragenyx research at https://www.ultragenyx.com/patients/tio/, NCBI research at https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7025948/;https://www.jstage.jst.go.jp/article/endocrj/62/9/62_EJ15-0275/_pdf/-char/en/. Prevalence data as of 2020.

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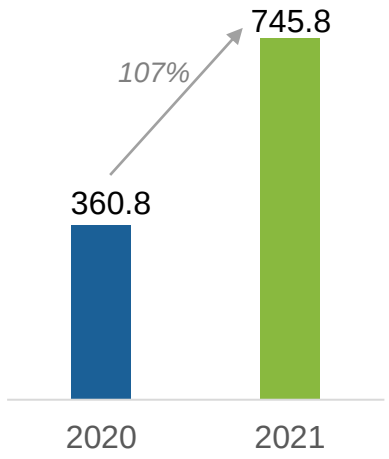
Financial Review

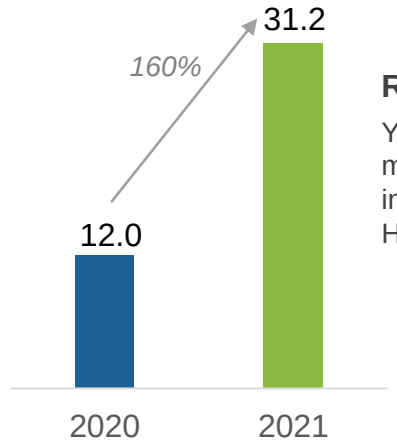
2021 Financial Highlights

RMB Million



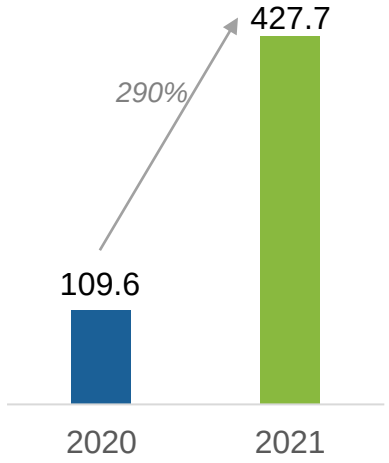
Cash Balance

YoY increase of RMB 385.0M, primarily attributed to our pre-IPO financing in May 2021 and the initial public offering



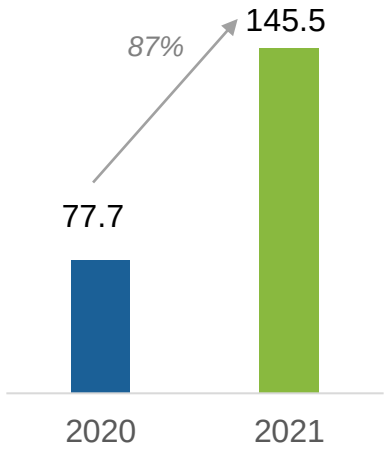
Revenue

YoY increase of RMB 19.2M mainly attributable to the increase of sales from Hunterase® and Nerlynx®



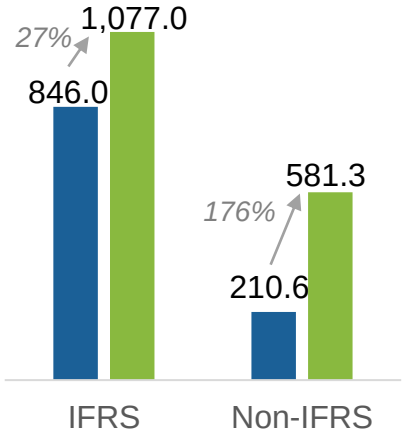
R&D Expenses

YoY increase of RMB 318.0M primarily attributable to increased payments made to our licensing partners, increased R&D employee costs and other testing and clinical trial expense



Administrative Expenses

YoY increase of RMB 67.8M, primarily attributable to: 1) increased staff costs due to headcount increase and new grant of share-based payments, 2) increased professional service fees as a result of the increase in relevant professional service fees with regard to our financing activities and business development activities, 3) increased listing expenses



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

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Outlook

Upcoming Key Milestones and Strategic Imperatives

We expect in the next two years:

As of Dec 2021, we have cash balance of RMB 746M

2022

CAN106 – Ph1 SAD full data presentation at major conference in 2H

CAN106 – initiate Phase 1b/2 trial in PNH in China in 1H

CAN108 – Establish special early access program of in designated hospitals in Boao (Hainan Province); initiate ALGS NPP in HK; dose first patient in BA Phase 2 study in China

CAN201 – Preclinical PoC mid 2022 and Pre-IND meeting with FDA in 2H

Release initial PoC gene therapy data at industry conference in 1H

Open **US-based Gene Therapy R&D center** in 2H

2023

CAN008 –Phase 2 interim readout in 2023

CAN108 – Potential approval for ALGS in China

CAN108 – Potential NDA filing for PFIC in China

CAN201 – IND filing to US FDA and plan to start trials in 2024

CAN202 – Potential IND filing to US FDA in 2024

DMD– Non-human PoC and announce pre-clinical lead candidate in DMD

CANbridge Innovative AAV Platform– Potentially establish in vivo PoC

Strategic Imperatives

Rare Disease Leadership

- Further solidify our leadership in the China's rare disease ecosystem
- Continue to build next generation global rare diseases franchise

Partnership and Collaboration

- Maximize value creation through partnership and collaboration
- Dedicate efforts to developing gene therapies

In-house Infrastructure

- Build fully integrated capabilities with in-house drug research, development and manufacturing infrastructure in global and Greater China markets

Abbreviations: NPP = Name Patient Program

Q&A

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THANK YOU

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Contact:
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Appendix

Income Statement

RMB'000	Year ended December 31 2021	2020
Revenue	**31,161**	**12,032**
Cost of sales	(12,385)	(5,154)
Gross profit (IFRS Measure)	**18,776**	**6,878**
Other income and gains	13,402	1,359
Selling and distribution expenses	(100,748)	(51,008)
Administrative expenses	(145,517)	(77,716)
Research and development expenses	(427,658)	(109,642)
Fair value changes of convertible redeemable preferred shares	(462,436)	(591,385)
Fair value changes of convertible loans	-	1,689
Fair value changes of derivative financial instruments	34,454	(20,746)
Other expenses	(4,200)	(1,599)
Finance costs	(3,079)	(3,873)
Loss before tax (IFRS Measure)	**(1,077,006)**	**(846,043)**
Adjustments to Non-IFRS measure	(495,674)	(635,427)
Adjusted loss for the period* (Non-IFRS Measure)	**(581,332)**	**(210,616)**

Revenue

YoY increase of RMB 19.2M mainly attributable to the increase of sales from Hunterase® and Nerlynx®

Research and Development Expenses

YoY increase of RMB 318.0M primarily attributable to increased payments made to our licensing partners, increased R&D employee costs and other testing and clinical trial expense

Administrative Expenses

YoY increase of RMB 67.8M, primarily attributable to:

- increased staff costs due to headcount increase and new grant of share-based payments
- increased professional service fees as a result of the increase in relevant professional service fees with regard to our financing activities and business development activities
- increased listing expenses

Loss for the Year

- IFRS loss for the year was RMB 1,077,006M
- 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

Balance Sheet

RMB'000	Year ended December 31 2021	2020
Property, plant and equipment	9,564	4,026
Right-of-use assets	19,978	11,544
Intangible assets	51,269	179,743
Total Non-current Assets	**80,811**	**195,313**
Inventories	13,448	553
Trade receivables	9,141	7,040
Prepayments, other receivables and other assets	43,307	22,648
Cash and cash equivalents	745,815	360,804
Total Current Assets	**811,711**	**391,045**
Total Assets	**893,443**	**586,358**
Trade payables	43,607	46,713
Other payables and accruals	103,423	33,557
Interest-bearing bank and other borrowings	30,868	22,314
Lease liabilities	7,882	5,519
Total Current Liabilities	**185,780**	**108,103**
Convertible redeemable preferred shares	-	2,167,121
Interest-bearing bank and other borrowings	-	11,645
Lease liabilities	13,351	7,417
Other non-current liabilities	-	1,456
Derivative financial instruments	-	36,472
Total Non-current Liabilities	**13,351**	**2,224,111**
Total Liabilities	**199,131**	**2,332,214**
Total Equity	**693,391**	**(1,745,856)**

Note: 1. Cash burn rate refers to the average monthly net cash used in operating activities, which includes research and development expenses, and capital expenditures.