CANbridge Pharmaceuticals

2023 Interim Result Presentation

Aug 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

CANbridge Today

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

2 Marketed Rare Disease Product

- MPS II — Hunterase

- ALGS in Mainland China* — LIVMARLI

4 Late-stage Clinical Programs

- Biliary Atresia — LIVMARLI
- PNH — CAN106
- Gaucher Disease — CAN103
- Glioblastoma — CAN008

3 Anticipated Registration Filing/Approval in Next 12 Months

- PFIC in Mainland China — LIVMARLI
- ALGS in Hong Kong — LIVMARLI
- ALGS in Taiwan — LIVMARLI

To launch in 1Q 2024

Well-established Commercialization Infrastructure

Launched Products

Hunterase

- Hunterase has entered into **109** cities' commercial insurance program ("Huiminbao") as of June 30, 2023
- **72%** of Hunterase treated patients are covered by commercial insurance
- Expected **NRDL inclusion** in **2024** and **10** newly expanded target cities with commercial insurance coverage

Livmarli

- Expected **NRDL inclusion** in **2024** and **4** newly expanded target cities with commercial insurance coverage
- Launch of Livmarli-ALGS in China in **Q1 2024**
- Dedicated sales team of 12 professionals support early launch to cover **98 hospitals** and **300** healthcare physicians

Expect 5 new launches in 5 years,
including CAN103, CAN108-PFIC, CAN108-BA, CAN106 and CAN008

Established Commercial Infrastructure in Greater China

Marcelo Cheresky
Chief Commercial Officer

- **20+ years** of experience in leading emerging market teams, establishing marketing and commercial operations, and regional country management
- **Deep experience in successful launches** of multiple products globally including Eloctate, Alprolix, Thyrogen, Cerezyme, etc.

Synageva · ultragenyx · SANOFI · genzyme

Fannie Man
China Commercial Head / GM of HK and Macau

- Responsible for the commercial operations in Mainland China
- **17+ years** of commercial experience in various companies including BMS, AbbVie and GSK, where she contributed in launching numerous key oncology, hematology, hepatology and respiratory products

Bristol Myers Squibb · abbvie · gsk GlaxoSmithKline

Commercialization team of 40+
experienced professionals

Broad Geographical Coverage in Mainland China

	Before NRDL	After NRDL
Covered Province #	22	30
Covered City #	~50	~180
Covered Hospital #	~300	~1,100
Covered KOL #	~1,500	~5,500
Covered Population # Mn	~550	~1,130

Established commercial infrastructure in HK and Taiwan to drive robust sales

Access to GMP Manufacturing Capability for Multiple Modalities

- *7 CMC staff in China and the U.S.*
- *Secured manufacturing capacity with third-party collaboration partners*
- *In-house process development lab in the U.S.*
- *Well-established quality control team in China and the U.S.*

Abundant manufacturing capacity

Solid partnership with Wuxi

Preferred access to the Wuxi's global manufacturing capacity across all modalities (ERT, mAb, Gene therapy)

WuXi Biologics Global Solution Provider

WuXi ADVANCED THERAPIES 药明生基

WuXi facilities

GC Pharma

US process development lab

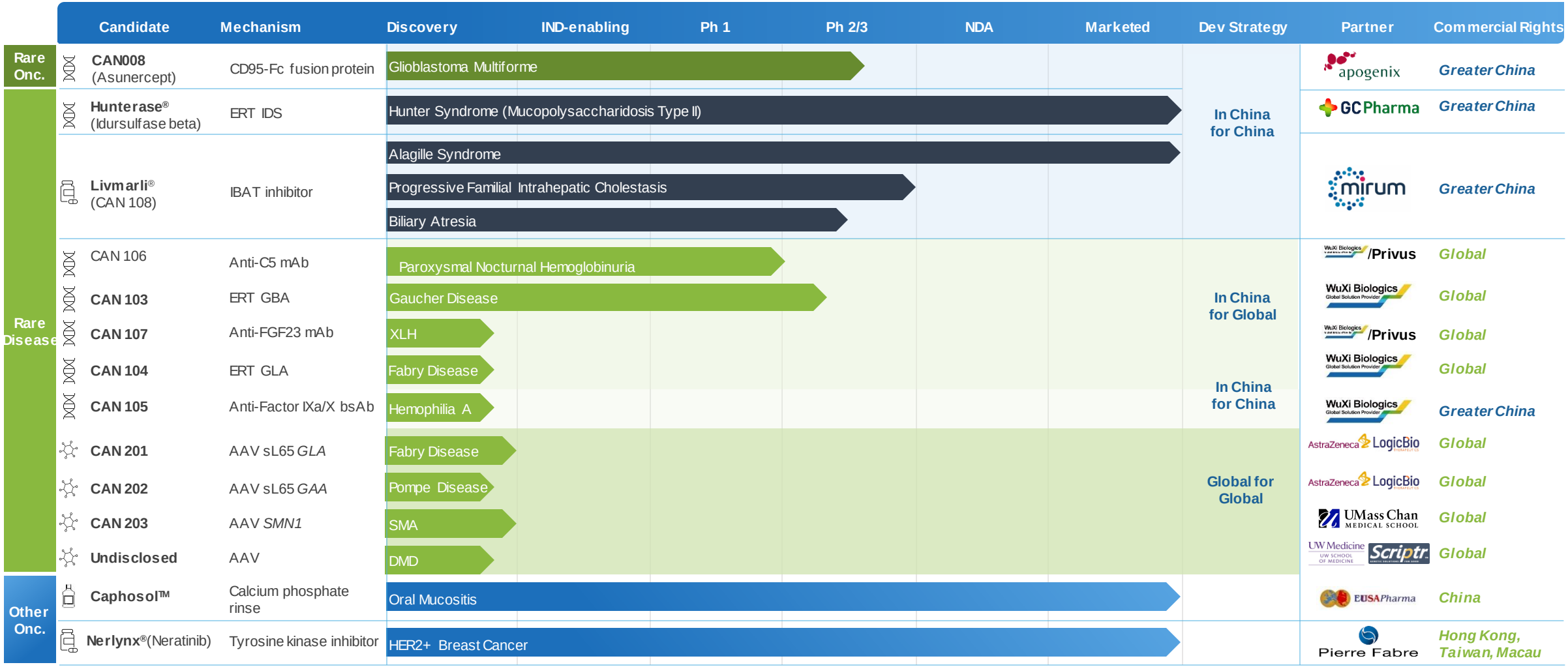
Total 24,500 sq. ft. (up to 90 FTEs), including AAV process lab (up to 50L scale); AAV analytical lab; Research discovery lab

AstraZeneca LogicBio THERAPEUTICS

AAV process development lab in Greater Boston, US

Our Comprehensive and Diversified Pipeline

CANbridge holds global rights to 8 out of 14 assets, spanning biologics, small molecules, and gene therapy, targeting most prevalent rare diseases and oncology indications, with proven mechanisms and significantt market potential



Pipeline Targets Diseases with $15 Billion Potential

Commercial Rights	Pipeline	Indications	Prevalence	Global Sales
China	Hunterase®	MPS II	8k	>500 M
	Livmarli (CAN108)	Alagille Syndrome	10k	75 M
		PFIC	5k	>25 M
		Biliary Atresia	6.5k*	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.

Note: targeted patient pool

Past and Upcoming Milestones

Two products planned for NRDL negotiation in 2023 and Multiple NDA filings expected by 4Q2024

	Livmarli	Hunterase	CAN106	CAN008	CAN103	CAN203
1H 2023	Approved for marketing in mainland China	Identified 739 patients, expanded to 109 cities, covering 586m pupolation	Released positive Phase 1b data	Phase 2 Enrollment completed in Q1 IDMC[1] completed an interim analysis and review of Phase 2 study and recommended the study continue without any changes	initiated Part B in the Phase 1/2 Clinical trial	Data Presented at ASGCT[2] shows CAN203 is able to achieve superior potency, efficacy and safety in mice with SMA compared to the benchmark vector
Upcoming	**H2 2023** – NRDL negotiation **H2 2023** – ALGS Approval in TW/HK **H2 2023** – BA Interim readout **H2 2024** – PFIC Approval in mainland China/Taiwan	**H2 2023** – NRDL negotiation **2023/24** – keep enhancing commercial insurance entrance to more cities	**H2 2023** – Phase 2 patient enrollment **2025** – potential approval by China CDE in 2025	**1H 2024** – plans to report data from the Phase 2 clinical trial **2024** – potential NDA filing in 2024	**End of 2024** – plan to file NDA	We continue to advance our novel second-generation gene therapy

1：IDMC, Independent Data Monitoring Committee,

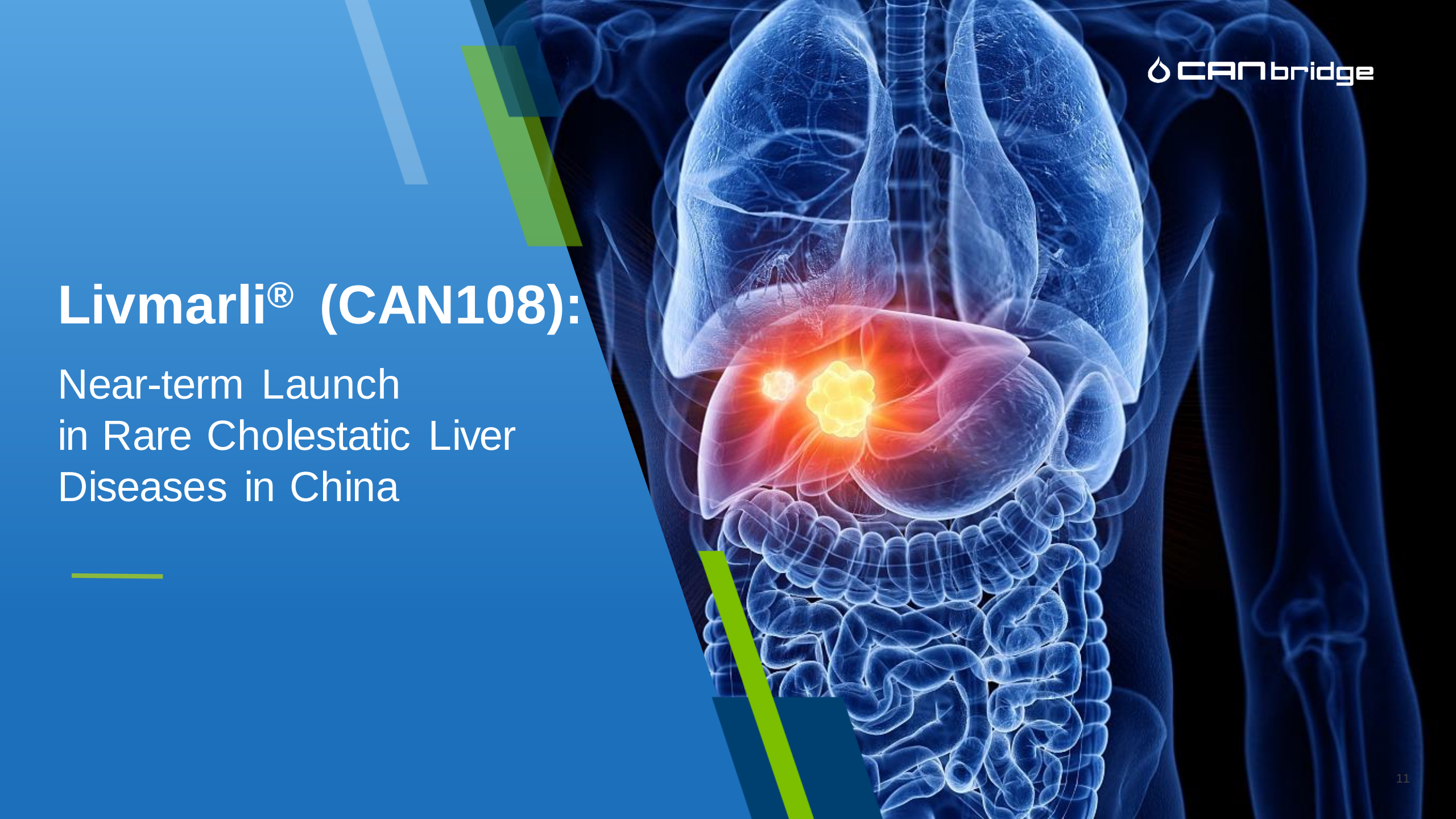
2：ASGCT, American Society of Gene and Cell Therapy Annual Meeting

CANbridge

Pipeline Portfolio Update

Livmarli® (CAN108):

Near-term Launch in Rare Cholestatic Liver Diseases in China



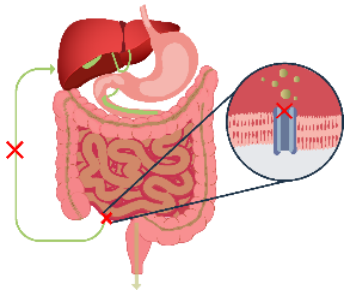
Livmarli – IBAT Inhibitor for Rare Cholestatic Liver Diseases

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

- **Received China NMPA marketing approval for ALGS** in 1H 2023
- **Filed Hong Kong/Taiwan FDA** for **ALGS**, with estimated approval by the end of 2023
- 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+

Mechanism of Action

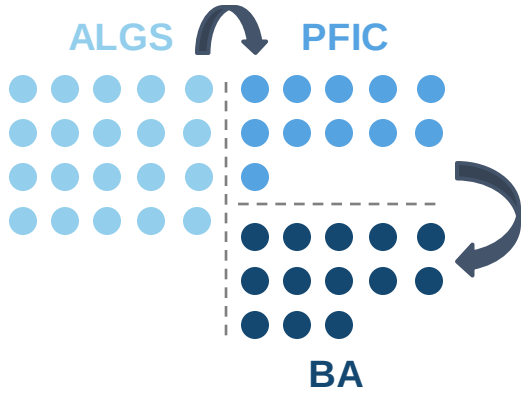


1. IBAT is primarily responsible for recycling bile acids from the intestine ileum back to the liver
2. Elevated bile acids damage the liver and lead to cholestatic liver disease
3. Livmarli is designed to inhibit IBAT in the ileum and result in more bile acids being excreted in the feces, leading to 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



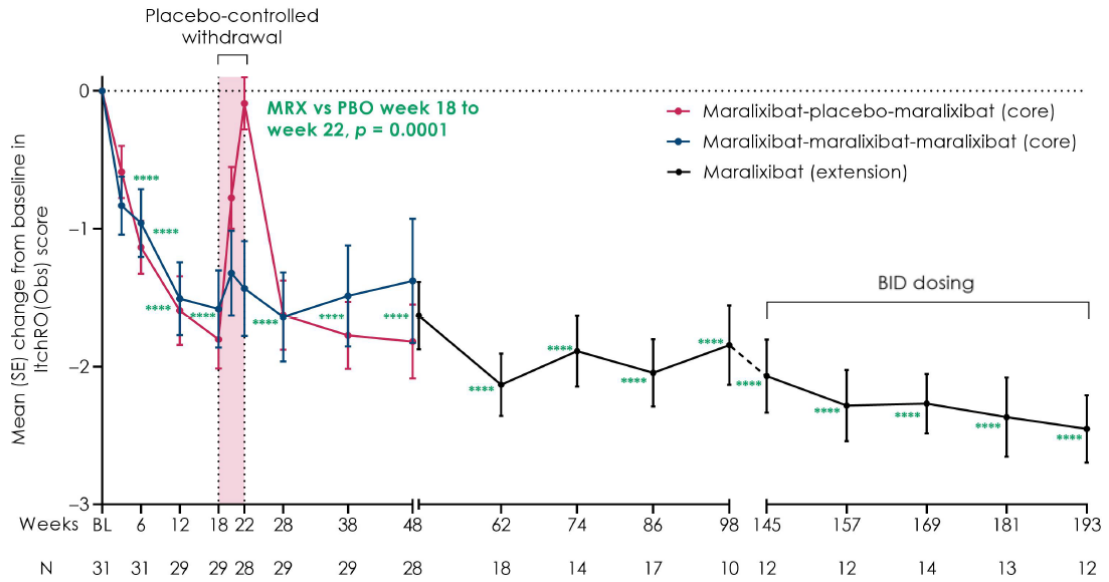
More ~22,000 targeted patients with ALGS, PFIC, BA in China

● Represent 500 patients

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

In China for China | In China for Global | In Global for Global

Statistically Significant, Clinically Meaningful, and Sustained Improvement in Pruritis



Change from baseline, **** $p \leq 0.0001$ (overall population)

- **Robust results from the pivotal LUM001-304 study, meeting the efficacy endpoint, improvement in pruritus, and long-term treatment benefit**
- **The results of the 4 supportive studies support the pivotal study efficacy results and effective dose**
  - The effect of maralixibat treatment on all efficacy parameters was maintained after Week 48 up to Week 240.

Safety Data of LIVMARLI in ALGS Includes 5 Years of Follow-up

Events observed over 5% patients	Number of events per 100 person-years
Diarrhea	41.6
Abdominal pain	38.6
Vomiting	19.8
Nausea	2.9
Fat-soluble vitamin deficiency	11.1
Transaminase increased	6.9
GI bleeding	3.8
Bone fractures	3.3

- **Population Exposure Status:** Maralixibat has been studied in > 1600 subjects, including more than 180 pediatric or adult subjects with cholestatic liver disease. Of 119 children with cholestatic liver disease treated for up to 5 years, 86 had ALGS.
- **Common Adverse Events:** In the ALGS pool, events of diarrhea and abdominal pain were the most frequently reported AE in subjects exposed to maralixibat and placebo.
- **Serious Adverse Events:** in the ALGS pooled population, no subjects in the majority of maralixibat clinical studies experienced SAE and no deaths were reported.

Adapted from Mirum Investor Communication

Long-term Data Emerging Suggests Improvement in Event-Free Survival and Growth

Maralixibat-treated patients with Alagille syndrome (ALGS) demonstrate improved event-free survival in a natural history comparison with patients from the GALA database: Application of real-world evidence analytics

GALA
The Global ALagille Alliance Study

Bettina E. Hansen, PhD[1,2]

and Binita M. Kamath, MBBChir[3] on behalf of The GALA Study Group

[1]Department of Epidemiology and Biostatistics, Erasmus MC, Rotterdam, The Netherlands
[2]Toronto Center for Liver Disease, University Health Network and the Institute of Health Policy, Management and Evaluation, The University of Toronto, Toronto, Canada
[3]The Hospital for Sick Children and the University of Toronto, Division of Gastroenterology, Hepatology, and Nutrition, Toronto, Canada

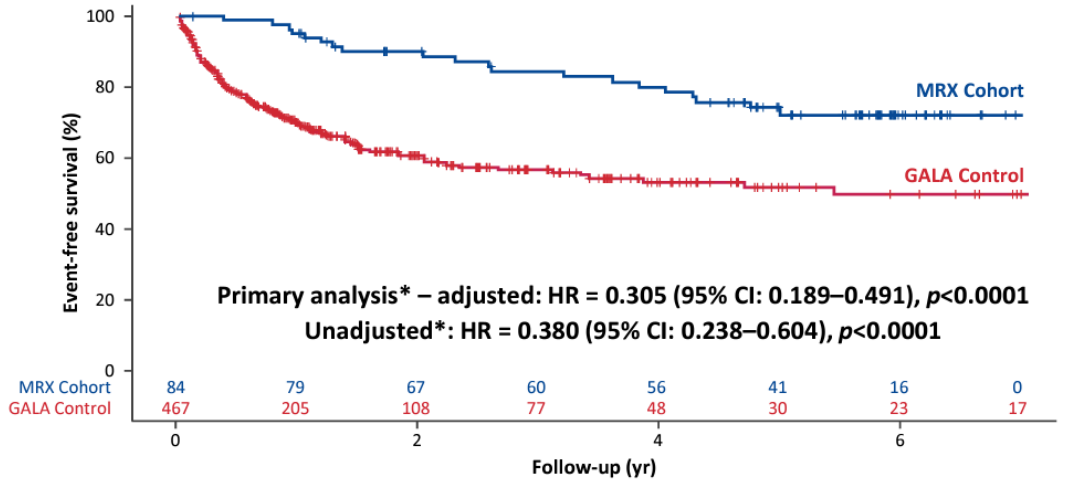
- Cohort of 84 patients treated with maralixibat compared with an external matched control cohort of 469 patients from the GALA* Database
- Events defined as: liver transplantation; biliary diversion surgery; liver decompensation event; or death.

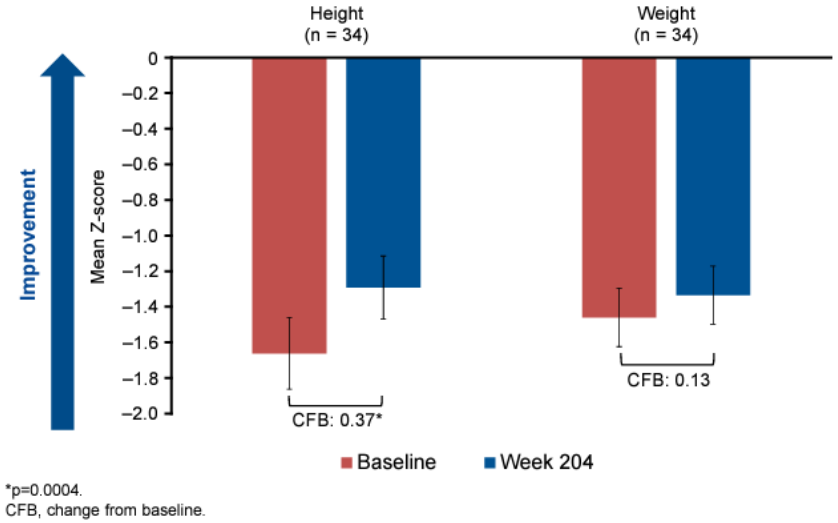
Maralixibat improves growth in patients with Alagille syndrome: A 4-year analysis

Binita M Kamath,[1] Douglas B Mogul,[2] Marshall Baek,[2] Tiago Nunes,[3] Pamela Vig[2]

[1]Division of Gastroenterology, Hepatology & Nutrition, Hospital for Sick Children and University of Toronto, Toronto, ON, Canada; [2]Mirum Pharmaceuticals, Inc., Foster City, CA, USA; [3]Mirum Pharmaceuticals, Inc., Basel, Switzerland

- Patients with ALGS typically suffer significant growth deficiency
- Mean height Z-score significantly increased from Baseline to Week 204 in ALGS patients treated with maralixibat



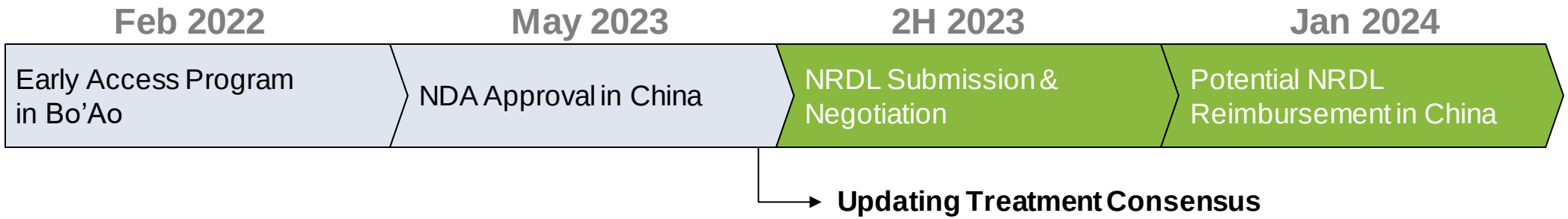


*p=0.0004.
CFB, change from baseline.

Adapted from posters and presentations at 2022 ESPGHAN Annual Meeting. GALA: Global Alagille Alliance

Commercial Launch of LIVMARLI in Reimbursed Market in Jan 2024

China market potential: $100-150 million in ALGS and $200-250 million for three indications combined



10,000 potential ALGS patients in China

~450
Current diagnosed patients

~350
Current addressable patients

CANbridge Sales Team of 12 dedicated to support early launch in 2024 to cover :

Target Hospitals: 98

Target Healthcare Physicians: 300

Anticipated approval and launch in Taiwan and Hong Kong between 2H 2023 and 1Q 2024

NRDL = national reimbursement drug list

In China for China | In China for Global | In Global for Global

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

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 739 identified patients

- **72** patients newly identified in 2023
- **263** patients newly identified in 2022
- **195** identified in 2021 since launch in May 2021
- **209** registered by patient group

Reimbursement Campaign

- **Hunterase has entered into 109** cities' commercial insurance program ("Huiminbao") as of end of June 2023, covering a populationof 586 million in China
- **72%** of Hunterase treated patients are covered by commercial insurance
- Reimbursement rate ranges from **20% to 90%**

CAN106

Clinically De-risked, Near Commercialization, Targeting Multiple Indications with Significant Market Potential

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

Novel, long-acting monoclonal antibody for the treatment of complement-mediated diseases, including PNH, myasthenia gravis (MG) and various other complement-mediated diseases that are targeted by anti-C5 antibodies.

Recent Highlights

- Completed first-in-human study conducted in Singapore in 2021 and **positive top-line data** reported
- **IND Approval** for the treatment of PNH in China in Jul. 2021
- **Orphan Drug Designation granted by FDA** for treatment of myasthenia gravis in Nov. 2022
- **Positive Phase 1b data in PNH** patients reported in Jun. 2023

Disease Overview

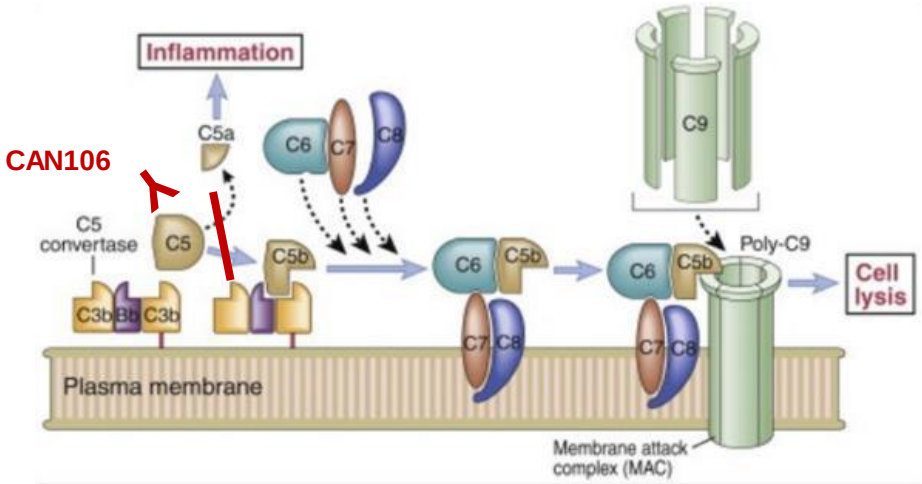
Paroxysmal nocturnal hemoglobinuria (PNH)

belongs to a group of fatal and rare disorders that occur when the complement system is dysregulated. In patients with PNH, the proteins that normally protect their red blood cells are not present, leaving these denuded cells susceptible to complement attack, which results in their destruction (hemolysis).

This leads to severe anemia, thromboembolism, gastrointestinal pain and dysfunction, fatigue, cardiac failure, pulmonary hypertension, renal impairment, and eventually, death.

PNH is an acquired genetic condition that can occur at any age across genders and race, but most commonly presents in adults in their 30s to 40s and continues for the life of the patient.

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

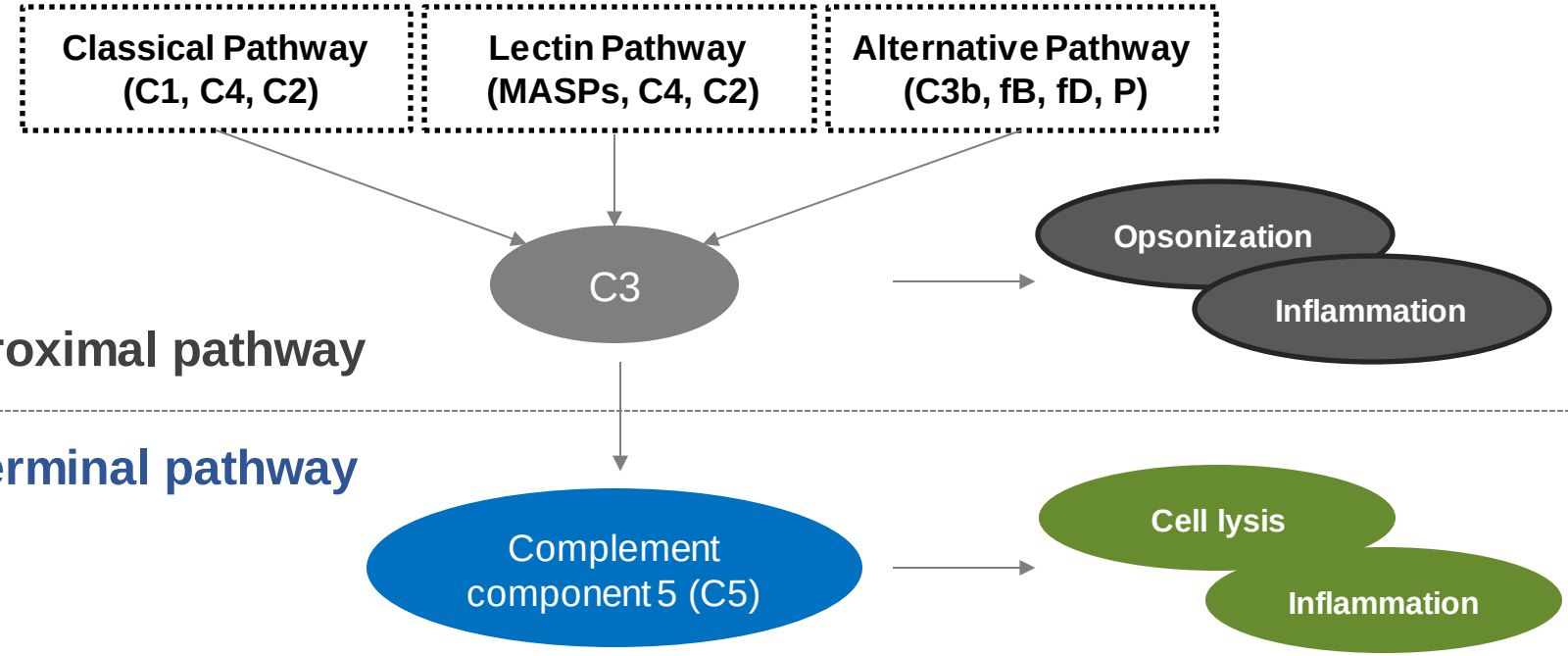
Source: Frost & Sullivan Analysis. Abbreviations: IBAT, ileal sodium-dependent bile acid transporter

C5 Iinhibition Remains the Current Standard of Care for PNH

Recent studies raise concerns about potential risks associated with proximal inhibitors in anti-complement treatment.

- Enhanced complement inhibition leads to heightened breakthrough hemolysis risk upon inhibition loss, due to increased susceptible PNH clones.
- Enzymatic activities upstream of the terminal pathway contribute to amplified breakthrough hemolysis risk with proximal inhibition.
- Loss of inhibition at the proximal level triggers exacerbated intra- and extra-vascular hemolysis severity, contrasting with terminal level inhibition loss.

Illustration of Complement Pathways



Therapeutics:
C3 inhibitor, fB inhibitor, fD inhibitor, etc

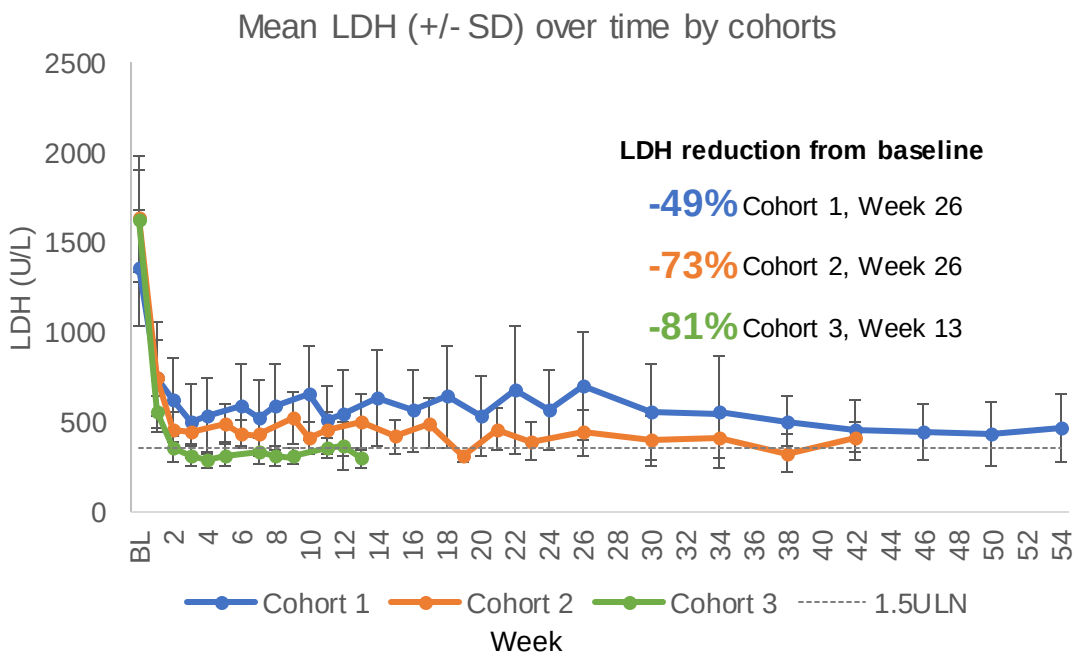
Severe breakthrough hemolysis
observed in clinic possibly led by transient loss of full inhibition when alternative pathway is targeted*

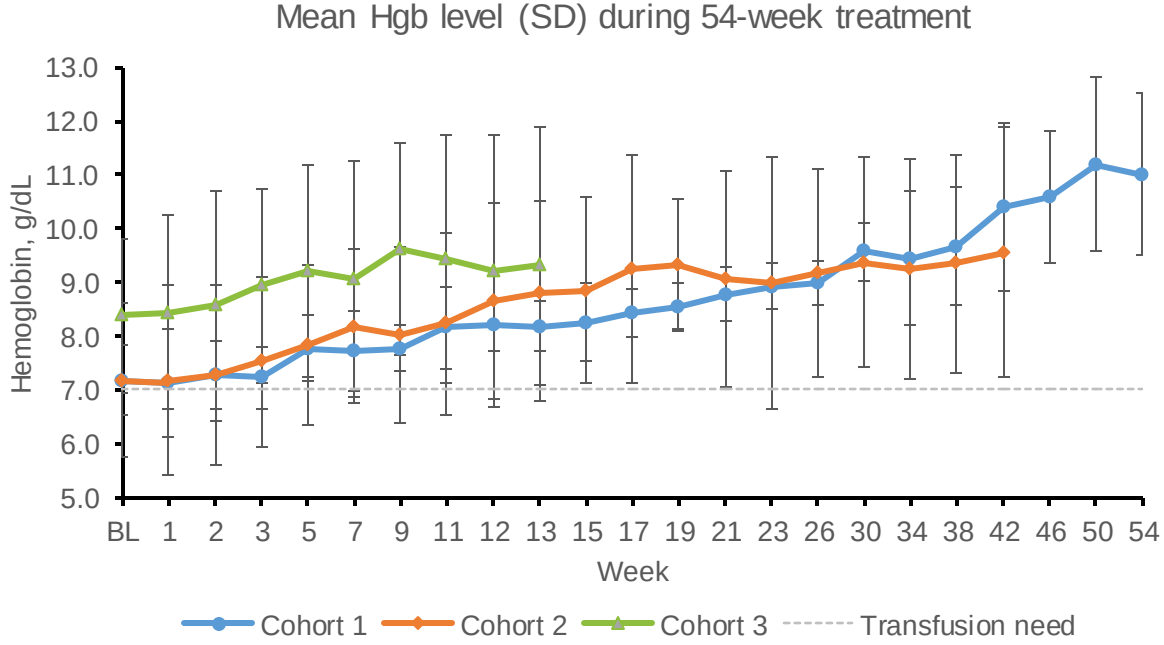
Therapeutics:
C5 inhibitors

PNH = Paroxysmal nocturnal hemoglobinuria *Risitano AM, et al. Immunological Reviews. 2022

Dose-dependent, Rapid and Substantial Effects on LDH and Hgb

Cohort 3: LDH, a biomarker of hemolysis, was reduced by 81% at Week 13. All subjects showed rapid and sustained LDH reduction, with 88% (7/8) subjects in Cohort 3 achieving LDH＜1.5 x ULN at least once after Week 3





Cohort	Maintenance Dose	Extension Dose (after Week 26)
1	20 mg/kg q4w	40 mg/kg q4w
2	40 mg/kg q4w	
3	80 mg/kg q4w	80 mg/kg q4w

Hgb levels improved across all cohorts.

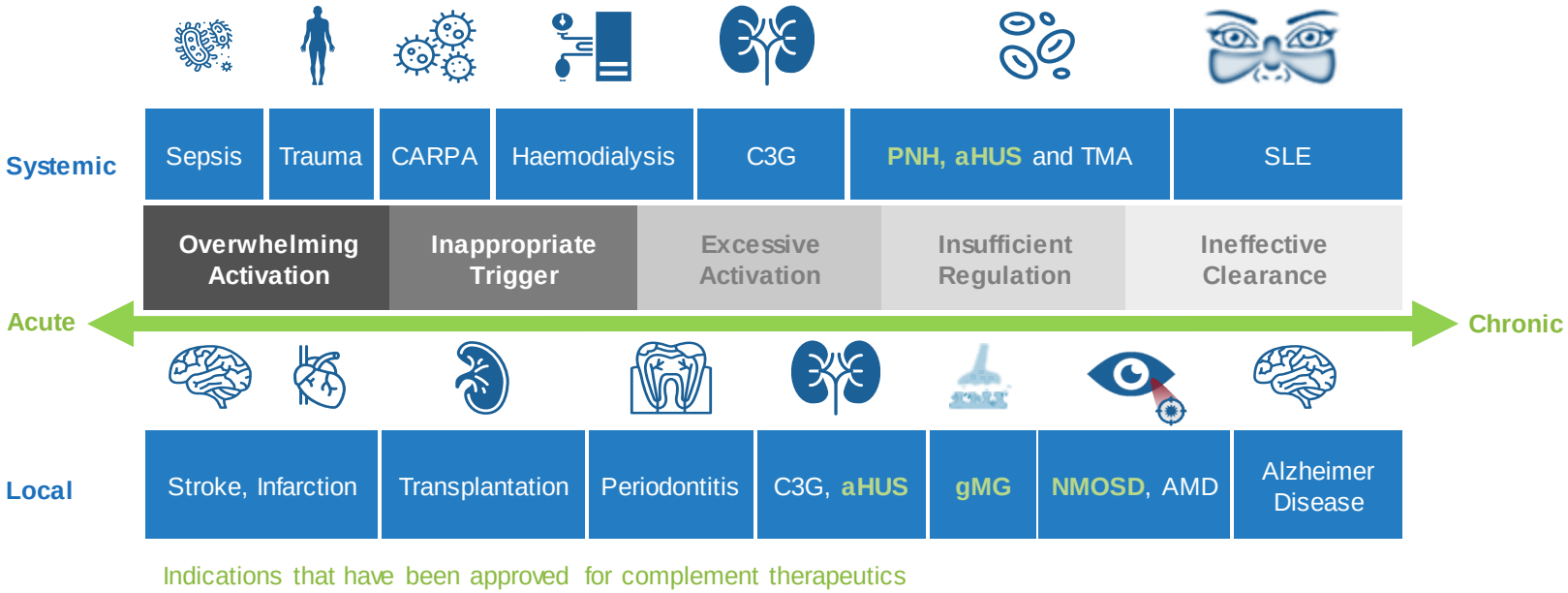
- Cohort1: Mean Hgb increase from baseline of 1.8 g/dL at Week 26 and 3.8 g/dL at Week 54.
- Cohort 2: Mean Hgb increase from baseline of 2.0 g/dL at Week 26 and 2.4 g/dL at Week 42.
- Cohort 3: Mean Hgb increase from baseline of 1.0 g/dL at Week 13.
- Two subjects in Cohort 3 at Week 13 achieved Hgb ≥12 g/dL in the absence of transfusion.

LDH=lactate dehydrogenase. Target range for hemolysis inhibition is LDH is < 1.5 x ULN = 351 U/L; Two breakthrough hemolysis events caused by COVID-19 were reported at Week 12 in Cohort 3, leading to transient elevations in LDH (> 2 x ULN) that recovered by Week 13.

CAN106 – Potential for Other Complement Disorders

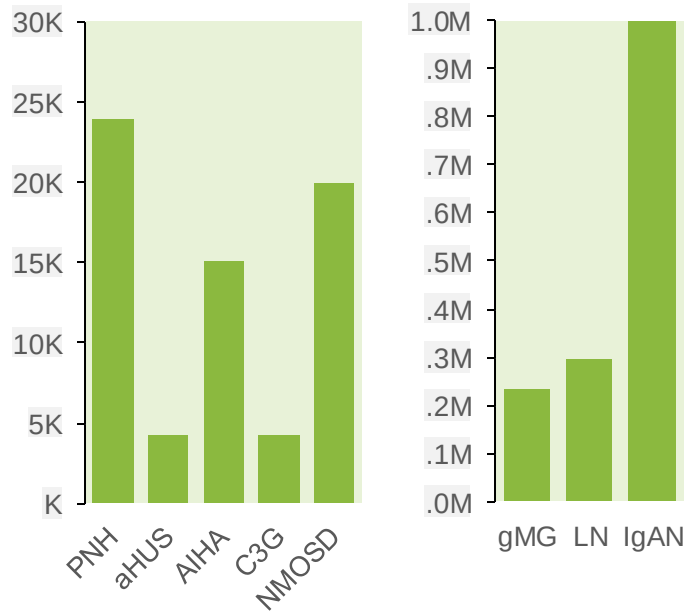
Potential "Pipeline in a Product". Initial development efforts focused on PNH treatment. Estimated global market revenue projections of >$9B in 20251

Potential Indications for Complement Therapeutics



Indications that have been approved 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

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CAN106 – Differentiated Follow-On Indication Strategy

CAN106's multi-indication potential, coupled with the limited access to the current available anti-C5 therapies in most parts of the world, allows for versatile indication expansion and go-to-market strategies to maximize its global commercial value

Soliris & Ultomiris Approval and Reimbursement Status[1]								
Soliris	*PNH*	✓	✓	✓	✓	✓	✓	✓
	aHUS	✓	✓	✓	✓	✓	✓	✓
	gMG	✓	O	✓	O	O	O	✓
	NMOSD	✓	O	✓	✓	O	O	✓
Ultomiris	*PNH*	✓	✓	✓	✓	✓	✓	✓
	aHUS	✓	✓	✓	✓	✓	✓	✓
	gMG	✓	X	O	O	O	O	✓
	NMOSD	X	X	O	O	O	O	O

Key: ✓ Approved, Reimbursed O Approved, Not Reimbursed X Not Approved

Diagnosed Prevalence for Select Complement-mediated Disorders[2]			
PNH	~5K	~8K	~1K
aHUS	~2K	~3K	~450
gMG	~66K	~102K	~13K
NMOSD	~13K	~6K	~4K

- Despite broad approval of existing C5 inhibitors, **patient access to Alexion's therapies** approved in gMG and NMOSD is still **limited, particularly in European and Rest-of-World markets**
- Access to anti-C5 therapies in developing countries is even **more limited or non-existent**
- Future CAN106 clinical development and commercialization plans can be **optimized to prioritize development in de-risked, high-value complement-mediated disorders in markets underserved by Soliris and Ultomiris**
- **Additional indications** where anti-C5 therapies are not approved yet are **available for exploration and expansion**

1. ClearView Healthcare Partners Analysis; Japan gMG reimbursement status inferred from previous reimbursement activities 2. CANbridge Internal Analysis

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

Neuromuscular Disorders

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 Brigham & 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:
Development in Newly Diagnosed GBM

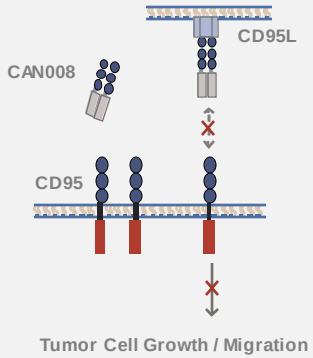
CAN008 – CD95-Fc Fusion Protein for Glioblastoma Multiforme (GBM)

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

Recent Highlights

- An independent data monitoring committee completed an interim analysis and review of the ongoing Phase 2 study of CAN008 and **recommended the study continue without any changes to the current trial design in July 2023.**
- 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

Mechanism of Action

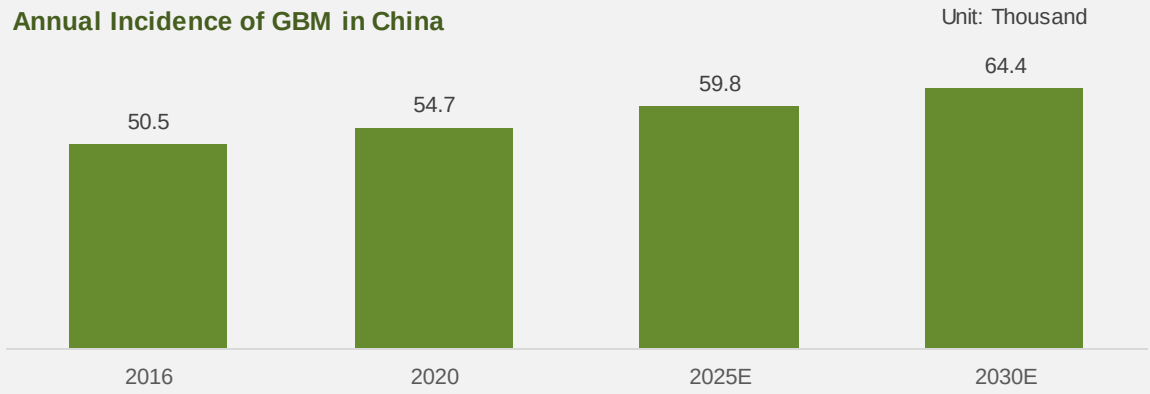


1. CD95L binds to its receptor, CD95, on the cell surface and induces the oligomerization of CD95, which triggers an intracellular signaling cascade that stimulates tumor cell growth and migration
2. CAN008 acts as a soluble receptor that specifically binds to CD95L and blocks the endogenous CD95 / CD95L 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, tumor treating field (TTF), bevacizumab (Avastin)

Epidemiology



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

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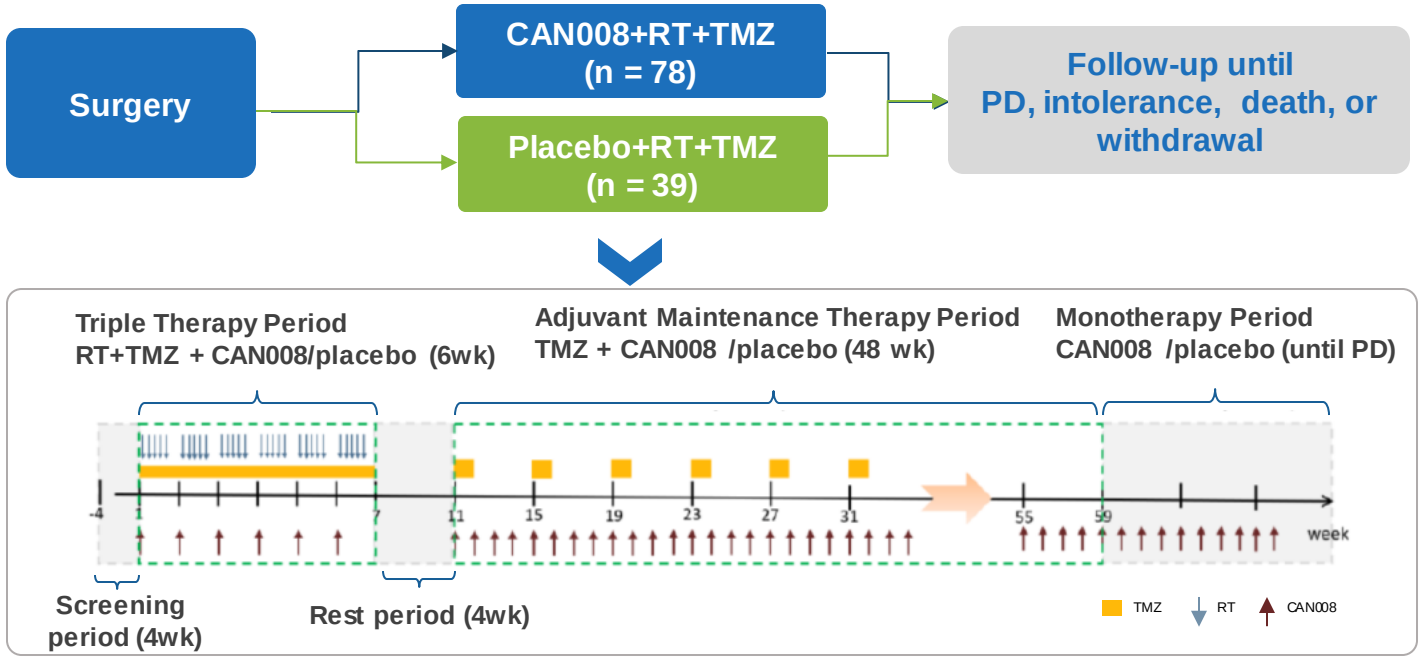
CAN008 – Ongoing Phase 2 Registrational Trial in Newly Diagnosed GBM

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



Primary endpoint

- Progression-free survival (PFS)

Interim Readout

- Progression of 37 cases

Next Catalyst

Key Development Timeline

Enrollment completion
in Q1 2023

Phase 2 interim analysis
in H2 2023

Phase 2 top-line data
in early 2024

Next-gen Gene Therapy Pipeline

CANbridge's Next-Generation Innovation and Process Development Facility in Burlington, Massachusetts

Proprietary Gene Therapy Technology Platform & Powerful Discovery Engine

We continue to invest in gene therapy as our next-generation technology development and expand our in-house R&D activity to generate promising leads for treatment of rare diseases

Research Collaborations with World-Renowned Investigators and Academic Institutions

Collaborations in Neuromuscular Disorders

UMass Chan Medical School

Investigators:
- **Dr. Guangping Gao**
- **Dr. Miguel Esteves**

UW Medicine, UW School of Medicine

Investigator:
- **Dr. Jeff Chamberlain**

Potentially best-in-class SMA GT
(Global rights secured)

*Novel **tissue-tropic AAV** capsid discovery*

Potentially best-in-class DMD GT

In-House AAV Gene Therapy Platform and Process Development Facility

Platform technology with guided AAV tissue targeting, such as CNS or muscles

AAV process development lab and pilot plants in Greater Boston

Strategic Collaborations with Innovative Industry Partners

LogicBio Therapeutics

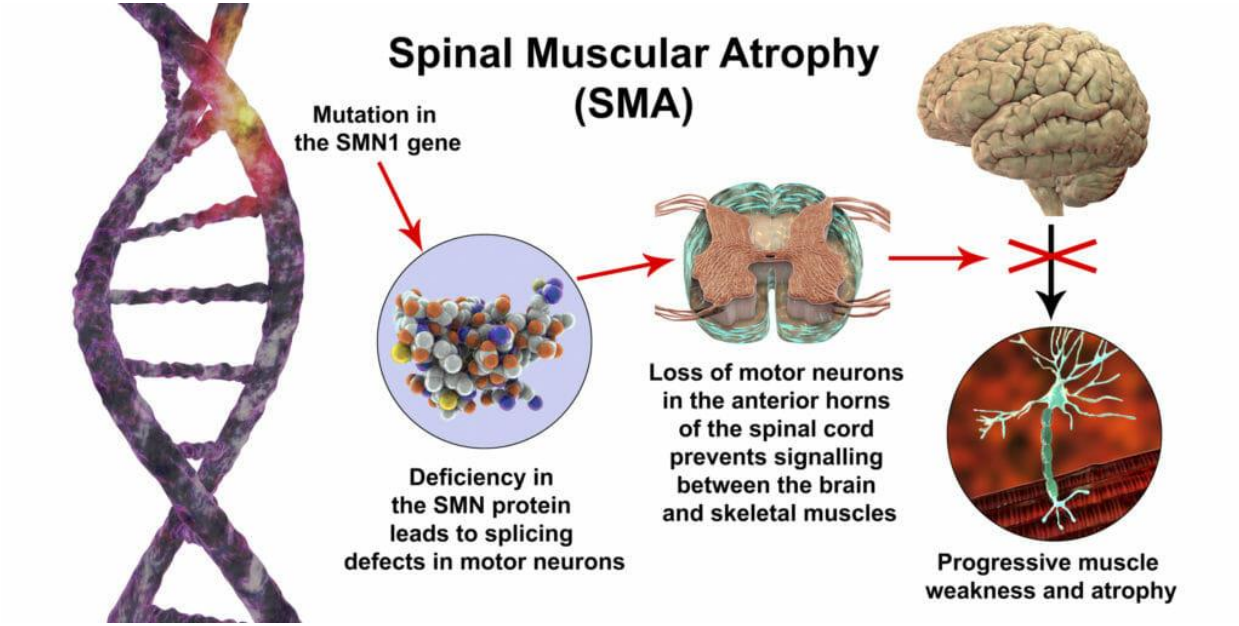
*Licensed worldwide rights to **liver-tropic capsid** for AAV gene therapy products to treat Fabry and Pompe diseases*

Scriptr™ Genetic Solutions for Good

*Exclusive worldwide rights to **proprietary RNA Assembly Technology** to develop next-gen GT for dystrophinopathies*

CAN203 – Second Generation Gene Therapy for Spinal Muscular Atrophy

SMA Pathophysiology Illustration



CAN203

- Second-gen gene therapy to potentially treat SMA type 1-3 (from infants to adult patients)
- Endogenous promoter: regulated, targeted tissue expression to avoid unwanted toxicity in liver and heart
- Codon optimized: enhanced tissue expression
- May achieve safer, more cost-effective treatment with lower doses compared to standard of care gene therapy
- Presented additional preclinical data at the premier academic conference 2022 and 2023 ASGCT.

Epidemiology

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

Unmet Need

- Patients with SMA over the age of two cannot be treated with the 1st-gen gene therapy Zolgensma®
- Black box warning of serious liver injury associated with Zolgensma®
- Limited access due to high price

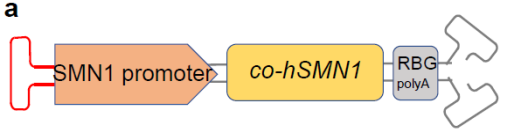
Source: Cowen Equity Research and SMA Foundation.

CAN203 - Preclinical Data Presented at 2022 and 2023 ASGCT

Head-to-head comparison between the 2nd-generation vector (CAN203) and the reference vector (designed similar to Zolgensma®): both IV* and ICV* injections demonstrate therapeutic advantages

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

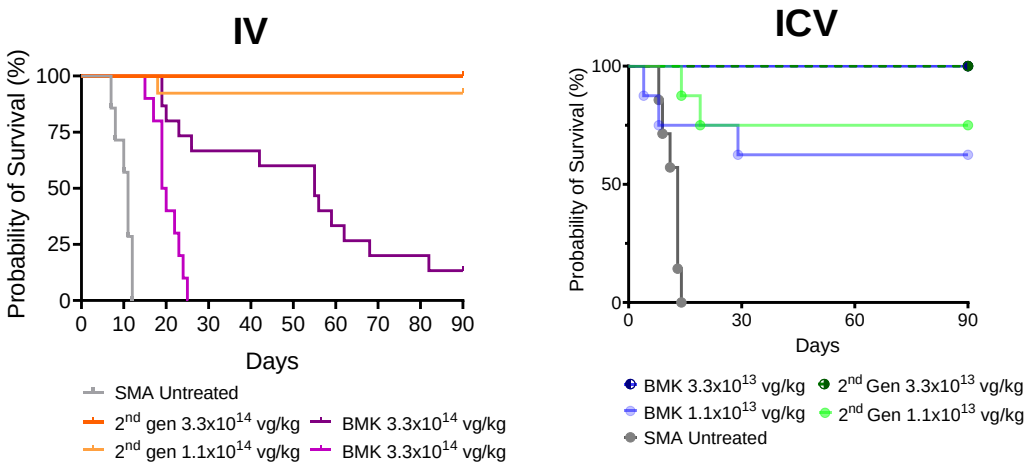
a



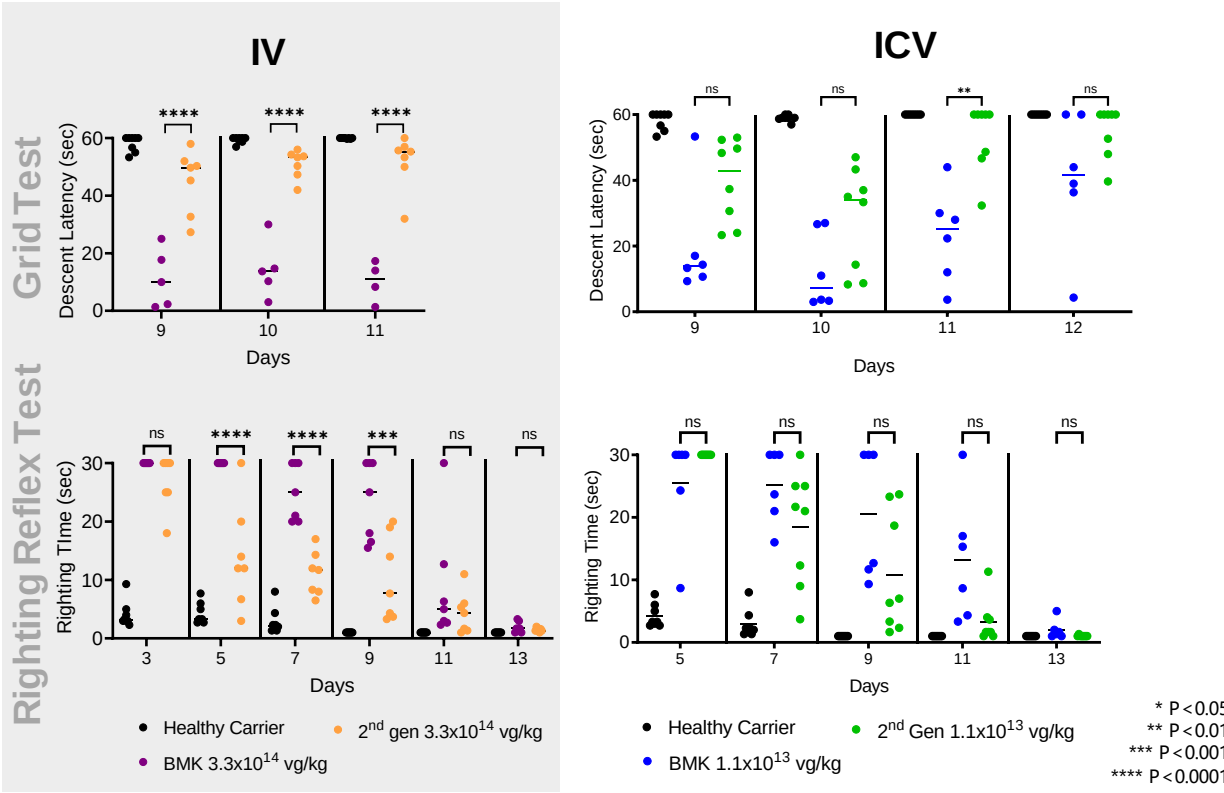
pAAVsc-*SMNp-co-hSMN1* (2nd gen vector)

Unlike the 1st gen benchmark vector that utilizes a ubiquitous promoter leading to non-specific high-level SMN expression across tissues, the 2nd gen vector (CAN203) utilizes an endogenous SMN1 promoter, enabling tissue-specific regulation of SMN protein expression.

Survival curve of vector-treated mice



2nd gen vector conferred significantly better restoration of motor function than the benchmark vector in SMA mice



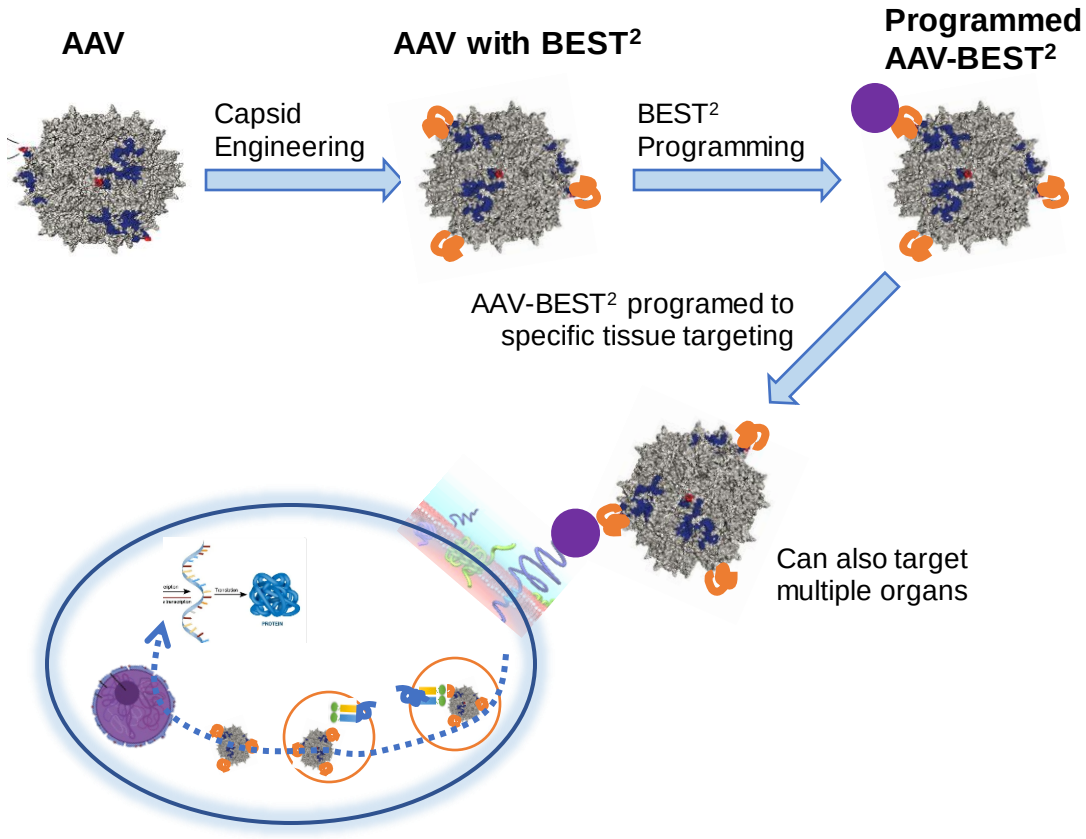
In vivo data demonstrates the superiority of the 2nd-generation vector in extending lifespan, improving motor function, and eliminating liver toxicity (data not shown)

IV: intravenous; ICV: intracerebroventricular

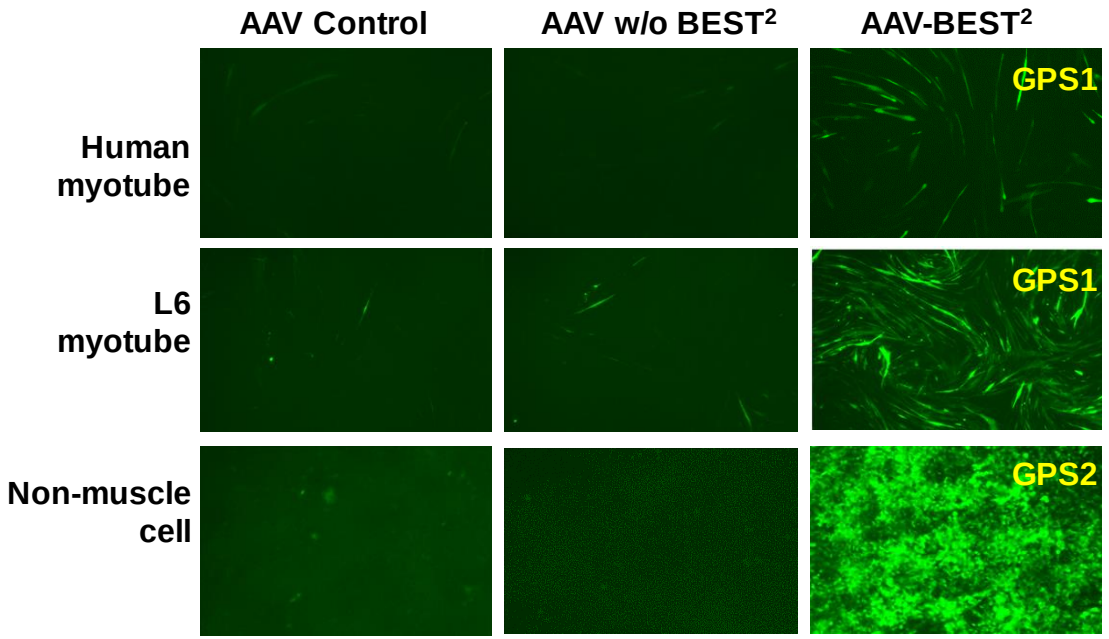
CANbridge Innovative AAV Capsid Platform: BEST²AAV

Addressing limitations of current AAV technology

CANbridge BEST² Tissue Specific Delivery Platform



PoC of BEST²AAV *in vitro* in Myotubes and Non-Muscle Cells



Additional Data

- BEST² demonstrated superior transduction to AAV, with similar transduction to MyoAAV*
- IVIG inhibited transduction of myotubes by AAV9 and MyoAAV, but not by BEST²

Note: Tabebordbar M et al, Cell 2021

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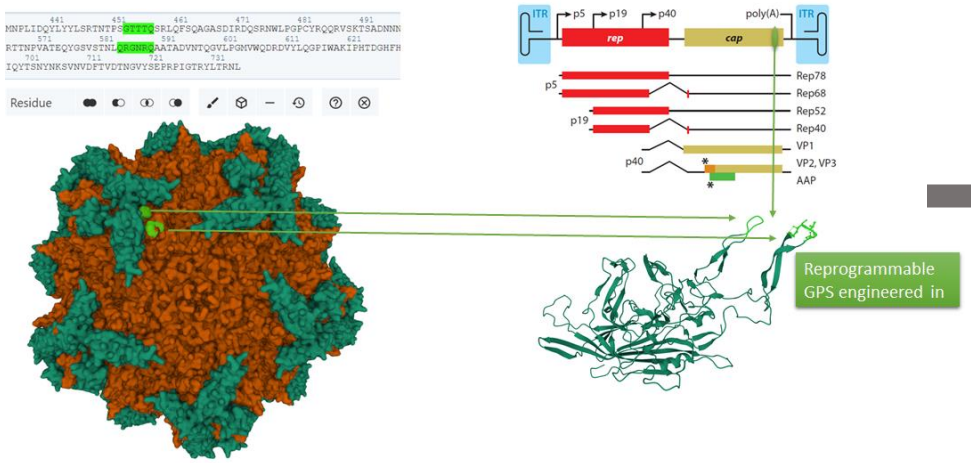
Comparison of CANbridge BEST^2 with Other Novel Capsid Approaches

	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**



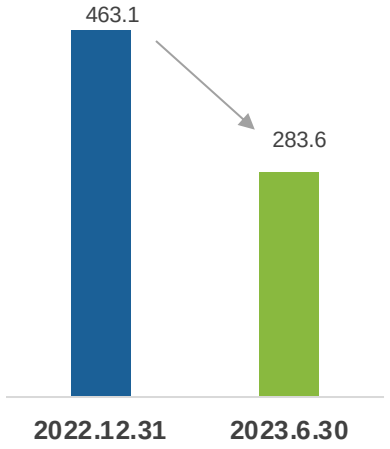
CANbridge AAV-BEST^2

- Small edits 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-like" 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 BEST^2 to further avoid NAbs for repeated dosing

Financial Review

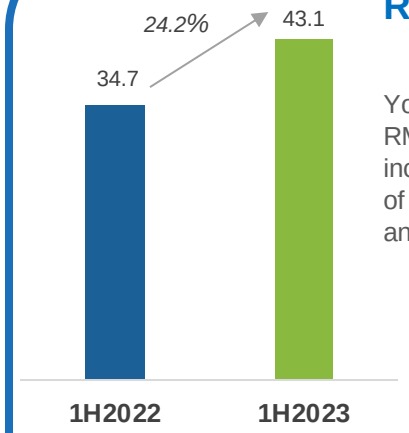
1H 2023 Financial Highlights

RMB Million



Cash Balance

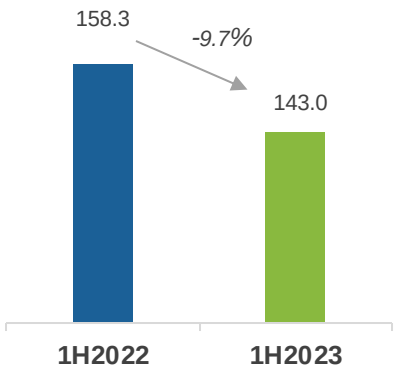
YoY decrease of RMB 179.5M, primarily attributed to net cash outflows used in operations



Revenue

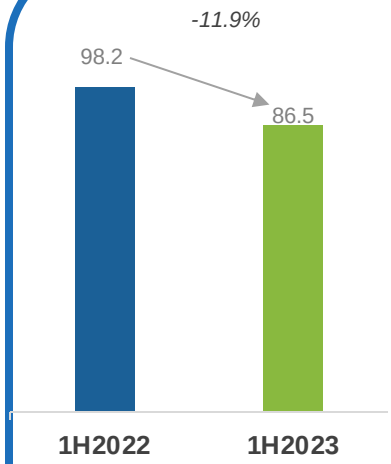
YoY increase of RMB 8.4M from increase of sales of Hunterase® and Livmarli®

R&D expenses including milestones payment



R&D Expenses

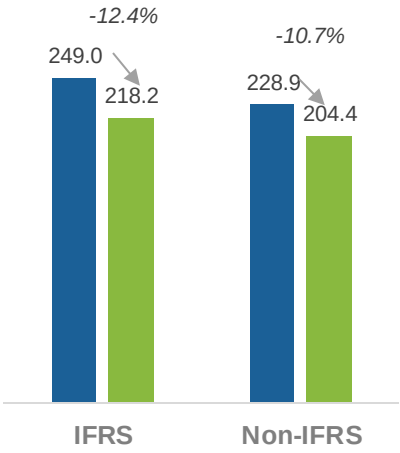
YoY decrease of RMB 15.3M. Due to lower upfront and milestone payments made to our licensing partners, lower technical service fees, partially offset by higher depreciation and amortization costs.



SG&A

YoY decrease of RMB 11.7M, decrease of our selling and distribution expenses was primarily due lower employee costs.

Decrease of our administrative expenses was primarily due to lower employee costs, office expenses and professional service fees, partially offset by the higher depreciation and amortization costs.



Loss for the Period

The Non-IFRS measure of the adjusted loss for the period is arrived at by adjusting the IFRS Loss for the Period excluding the effect of share-based payment expenses.

Q&A

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THANK YOU

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