

SUMMARY

This summary aims to give you an overview of the information contained in this document. As this is a summary, it does not contain all the information that may be important to you. You should read the entire document before you decide to [REDACTED] in the [REDACTED]. In particular, we are a biotechnology company seeking to [REDACTED] on the Main Board of the Stock Exchange under Chapter 18A of the Listing Rules on the basis that we are unable to meet the requirements under Rule 8.05 (1), (2) or (3) of the Listing Rules. There are unique challenges, risks and uncertainties associated with [REDACTED] in companies such as ours. In addition, we have incurred significant operating losses since our inception, and we expect to remain loss making in the near term. We had negative net cash flow from operating activities during the Track Record Period. We did not declare or pay any dividends during the Track Record Period and do not intend to pay any dividends in the near future. Your [REDACTED] decision should be made in light of these considerations.

There are risks associated with any [REDACTED]. Some of the particular risks in [REDACTED] in the [REDACTED] are set out in the section headed “Risk Factors” in this document. You should read that section carefully before you decide to [REDACTED] in the [REDACTED].

BUSINESS OVERVIEW

We are a China-based, rare disease-focused biopharmaceutical company founded in 2012 that is committed to the research, development and commercialization of biotech therapies. As of the Latest Practicable Date, we had developed a comprehensive pipeline of 13 drug assets with significant market potential, including three marketed products, four drug candidates at clinical stage, one at IND-enabling stage, two at preclinical stage and another three gene therapy programs at lead identification stage⁽¹⁾. Our products and product candidates target some of the most prevalent rare diseases as well as rare oncology indications, including but not limited to glioblastoma (GBM) and Mucopolysaccharidosis Type II (MPS II or Hunter syndrome). GBM represents 46.6% of the total incidence of brain cancer in China and has reached 54.7 thousand in 2020 with a CAGR of 2.0% from 2016 to 2020, and is expected to grow steadily to 59.8 thousand in 2025 and 64.4 thousand in 2030 at a CAGR of 1.8% and 1.5%, respectively. MPS II is the most common form of MPS disorders in East Asian countries. The prevalence of MPS II in Greater China reached 8,005 in 2020 and is estimated to reach 8,175 in 2030. CAN008, our Core Product, is a glycosylated CD95-Fc fusion protein being developed for the treatment of GBM. We are developing the other 12 of the drug candidates in our pipeline as of the Latest Practicable Date. As of the Latest Practicable Date, we had exclusive rights to 2 granted patents and 1 patent application for CAN008 owned by Apogenix, our collaboration partner. We are also conducting R&D works for other indications beyond GBM, which may be the subject of additional patent filings by ourselves in the short to mid-term.

(1) lead identification stage is when we are identifying the chemical that will interact with the target with the potential to treat the disease studied.

SUMMARY

WE MAY NOT BE ABLE TO SUCCESSFULLY DEVELOP AND/OR MARKET OUR CORE PRODUCT CANDIDATE, OR ANY OF OUR PIPELINE PRODUCTS.

OUR BUSINESS MODEL

We strategically combine global collaborations and internal research to build and diversify our drug portfolio. As the Chinese rare disease market rapidly expands, many international biopharmaceutical companies are interested in accessing this growing and untapped market but lack the local expertise. Leveraging our global collaborations and R&D capabilities, we believe we can serve as a gateway to China and a preferred partner for international biopharmaceutical companies. As of the Latest Practicable Date, our global partners include but not limited to Apogenix, GC Pharma, Mirum, Wuxi Biologics, Privus, University of Massachusetts Medical School (UMass) and LogicBio. In 2019, we in-licensed Hunterase® (CAN101) from an international biopharmaceutical company, GC Pharma, which is our first commercialized rare disease product to address unmet needs in China, supported by clinical validation and marketing authorization in over 10 countries worldwide by GC Pharma. We are working with our research partner UMass on sponsored research programs to develop gene therapy solutions for neuromuscular disorders, with the exclusive option to license-in the assets for development. We also seek to collaborate with China-based academic institutions. In addition, our experienced research team continues our efforts in identifying and developing drug candidates to further expand our portfolio. For example, our internal research team is developing gene therapy solutions for neuromuscular disorders. We leverage our commercialization capabilities to maximize the market potential of our drug candidates. We established key operation hubs in Beijing and Shanghai and offices in other locations in Greater China, and plan to expand to each of the key target provinces with local offices in mainland China. We are currently expanding our targeted, in-house commercialization team, which is expected to expand into over 300 members in the next five years.

Leveraging our experienced management team, a comprehensive product portfolio and an integrated platform with access to industry leading rare disease technologies, we believe we are well positioned to capture the vast rare disease market in China and globally.

SUMMARY

The following chart summarizes our portfolio and the development status of each drug asset as of the Latest Practicable Date:

| Product | Modality (Drug Category (Drug Category (Drug Category Administration Law) ⁽¹⁾) ⁽²⁾) | Mechanism | Indication | Discovery/Pre-clinical | IND-enabling | Ph I | Ph II/III | NDA | Marketed | Partner | Commercial Rights ⁽³⁾ | In-licensing Date | Granted Patents FN ⁽⁴⁾ | Patent Applications FN ⁽⁵⁾ |
|----------------------------------------------------|-------------------------------------------------------------------------------------------------------------|-------------------------------|--------------------------------------------------------------------------------------------------|-------------------------------------------------------------------------------|--------------|------|-----------|-----|----------|-------------------------------|------------------------------------------|---------------------------------------------------------------------------------------------------------------|-----------------------------------|---------------------------------------|
| CAN108 (Aumercet) [★] | Biologic (Cat.1 of Biological Drugs) | CD95-Fc fusion protein | Glioblastoma Multiforme ⁽²⁾ | Taiwan Phase I trial completed China Phase 2 trial initiated | | | | | | apogenix | Greater China | June 26, 2015 (with exclusive rights to develop, manufacture and commercialize) | 3 | 2 |
| Heritase [®] (CAN109) (Atrialfibrak beta) | Biologic (Cat.3 of Biological Drugs) | ERT diatomate-2-sulfate (IDS) | Hunter syndrome (Mucopolysaccharidosis type II) ⁽¹⁾ | China Phase 1 trial initiated | | | | | | GUC Pharma | Greater China | January 3, 2019 (with exclusive rights to develop and commercialize; commercialization commenced in May 2020) | 1 | 2 |
| CAN 108 (maralixibat) | Small Molecule (ALGS, Cat.5 of Chemical Drugs, PFC: Cat.2,4 of Chemical Drugs) | ASBT-inhibitor | Alagille Syndrome ⁽⁴⁾ Progressive Familial Intrahepatic Cholestasis ⁽⁴⁾ | China Phase 1 trial initiated China Phase 2 trial initiated | | | | | | Amgen | Greater China | April 28, 2021 (with exclusive rights to develop, manufacture and commercialize) | 2 | 5 |
| CAN 106 | Biologic (Cat.1 of Biological Drugs) | Anti-C5 mAb | Biliary Atresia ⁽⁴⁾ | Global (U.S., Europe and China) Phase 2 initiated | | | | | | WuXi Biologics / Privus | Greater China | January 7, 2019 (with exclusive rights to develop and commercialize) | 0 | 23 |
| CAN 103 | Biologic (Cat.1 of Biological Drugs) | ERT GBA | Paroxysmal nocturnal hemoglobinuria (PNH) and other Complement Disorders ⁽⁴⁾ | Singapore Phase 1 trial initiated China Phase 1 trial IND approved by NMPA | | | | | | WuXi Biologics | Global / Greater China | October 25, 2018 (with exclusive rights to develop and commercialize) | 0 | 2 |
| CAN 107 | Biologic (Cat.1 of Biological Drugs) | Anti-FGF23 mAb | Gaucher Disease ⁽⁴⁾ | China Phase 1 trial IND approved by NMPA | | | | | | WuXi Biologics / Privus | Greater China / Global ⁽¹⁾ | January 7, 2019/March 25, 2021 (with exclusive rights to develop and commercialize) | 0 | 2 |
| CAN 104 | Biologic | ERT GLA | X-linked hypophosphatemia | | | | | | | WuXi Biologics | Global | January 7, 2019 (with exclusive rights to develop and commercialize) | 0 | 0 |
| CAN 105 | Biologic | Anti-Factor DaxX hexab | Fabry Disease | | | | | | | WuXi Biologics | Greater China | January 7, 2019 (with exclusive rights to develop and commercialize) | 0 | 0 |
| Undiscovered ⁽⁶⁾ | Gene Therapy | AAV | Hemophilia A | | | | | | | University of Maryland System | Global | September 1, 2021 (sponsored rights to develop and commercialize) | 0 | 0 |
| CAN 201 | Gene Therapy | AAV ΔL65 GLA | Neuromuscular Disorders ⁽⁶⁾ | | | | | | | University of Maryland System | Global | April 26, 2021 (with exclusive rights to develop, manufacture and commercialize) | 0 | 3 |
| CAN 202 | Gene Therapy | AAV ΔL65 GNA | Fabry Disease ⁽⁶⁾ | | | | | | | LogicBio | Global | April 26, 2021 (with exclusive rights to develop, manufacture and commercialize) | 0 | 3 |
| Capbio ⁽¹⁾ | Medical Device ⁽¹⁾ (Class III Medical Device) | Calcium phosphate rinse | Oral Mucositis | | | | | | | TESA/Pharma | China | July 20, 2018 (commercialization commenced in October 2018) | 0 | 0 |
| Nerlynx [®] (Neratinib) | Small molecule (Cat.2,1 of Chemical Drugs) | Tyrosine kinase inhibitor | HER2+ Breast Cancer HER2+ Metastatic Breast Cancer | | | | | | | Pierre Fabre | Hong Kong, Taiwan, Mexico ⁽¹⁾ | February 24, 2011 (commercialization commenced in December 2010) | 12 | 7 |

★ Core Product █ Clinical trials performed by license partner ▨ Next Milestone

SUMMARY

Notes:

1. Greater China includes mainland China, Hong Kong, Taiwan and Macau.
2. After in-licensing, we have completed Phase 1 trial in Taiwan and obtained IND approval for the first line GBM Phase 2 clinical trial in China, for which we dosed the first patient in October 2021.
3. After in-licensing, we obtained a clinical trial waiver on the pivotal trial and the NDA approval for Humera[®] (CAN101) for MPS II from the NMPA in September 2020.
4. Mirum received the FDA approval for CAN108 for the treatment of cholestatic pruritus in patients with ALGS in September 2021. Mirum has also filed an MAA for ALGS for CAN108 with the EMA in September 2021. After in-licensing, we have started preparation of NDA for ALGS for CAN108 and expect to file NDA in Greater China in December 2021 based on data obtained by our license partner in global studies.
5. For BA, we are supporting the patient recruitment and clinical site management in China for a global Phase 2 clinical trial initiated by our license partner in the U.S. and Europe. IND approval was obtained from the NMPA in May 2021 and we plan to begin patient enrollment in China as part of the global Phase 2 trial in the first half of 2022.
6. After in-licensing, initiated a Phase 1 clinical trial for CAN106 in Singapore in February 2021 and obtained the IND approval from the NMPA for PNH for a Phase 1 study in China in July 2021.
7. Greater China rights granted from Wuxi Biologics. Worldwide excluding Greater China rights granted from Privus.
8. The Company obtained IND approval from NMPA in October 2021 for Gaucher Disease for a Phase 1 study in China. We plan to initiate patient enrollment in the first half of 2022.
9. Gene therapy programs at lead identification stage, including two programs (CAN201 and CAN202) licensed from LogicBio and one undisclosed program with exclusive option to enter into a licensing agreement with UMass (the program name is not available yet).
10. Caphosol[™] is an oral electrolyte solution and designated as a prescription medical device.
11. We have entered into a distribution agreement with Pierre Fabre pursuant to which Pierre Fabre appointed us as its distributor with exclusive rights to sell Nerlynx[®] (CAN030) for Pierre Fabre in Hong Kong, Macau, and Taiwan Pierre Fabre has the exclusive rights to develop and commercialize Nerlynx[®] (CAN030) in Greater China.
12. Patents were granted on each drug and patent applications were filed on each drug by the collaboration partners.
13. Each category refers to different registration pathway of a drug's approval process. For the drug assets not assigned a category, they are still at early stage and the Company has not yet decided the registration process in applying for the NDA. Each assigned category is referring to the following:
 - Cat.1 of Biological Drugs: Innovative therapeutic biologics not commercialized in China or overseas;
 - Cat.3 of Biological Drugs: Domestically or overseas marketed biological products;
 - Cat.2.4 of Chemical Drugs: Drugs with new indications containing known active ingredients that are not commercialized in China or overseas;
 - Cat.3 of Chemical Drugs: Generic drugs applied by domestic applicant, with an innovative drug that has been marketed overseas but not marketed domestically. Such drugs should be consistent with the quality and efficacy of the reference listed drug;
 - Cat.5.1 of Chemical Drugs: Domestic application for an innovative drug or a modified drug that has been marketed overseas. The modified drug should have obvious clinical advantages.

SUMMARY

OUR CORE PRODUCT

CAN008 CD95-Fc fusion protein for GBM

CAN008, our Core Product, is a glycosylated CD95-Fc fusion protein being developed for the treatment of GBM. We commenced a Phase 1 trial of CAN008 in combination with radiation therapy (RT) and temozolomide (TMZ) in patients with newly diagnosed GBM in September 2016 in Taiwan under the authorization of the Taiwan Food and Drug Administration (“TFDA”). A Phase 2 trial of CAN008 was completed by Apogenix AG (Apogenix) in recurrent GBM in Europe in September 2014. We completed the Phase 1 trial in Taiwan in September 2018 after 24 months of clinical research and development and results show CAN008 was generally well tolerated in patients with GBM. No dose-limiting toxicity was observed and no treatment-related serious adverse events were reported. We received the IND approval for CAN008 from the NMPA in March 2018 for a second-line Phase 2 trial and subsequently amended our IND application to a first-line Phase 2 trial based on the positive preliminary efficacy results obtained in the Phase 1 trial in Taiwan, which suggested the potential of CAN008 to become a standard-of-care treatment⁽¹⁾. We received the approval for a first-line Phase 2 trial in China on patients with GBM in April 2021 and dosed the first patient in China in October 2021. The Phase 2 clinical trial is designed to be multi-center, randomized, double-blind and placebo-controlled to investigate the efficacy and explore the correlation of different biomarkers with treatment outcome. We expect to commercialize CAN008 in China as a combination therapy with the standard of care for GBM (radiotherapy plus chemotherapy).

There are currently three targeted drugs for GBM marketed in China and seven being developed in China and worldwide. For details, see “Industry Overview – Glioblastoma Multiforme (GBM)”.

Core Product Candidate Development Process

Since in-licensing CAN008 from Apogenix in June 2015, we have led all R&D strategy and been primarily responsible for all clinical development activities related to CAN008 in Greater China, which include the biomarker study, the Phase 1 study and the Phase 2 study. We have engaged in substantial R&D work for more than 12 months on CAN008, primarily including:

- (a) From July 2015 to January 2016, we completed a biomarker study for expression of CD95L, the first of its kind in China, in over 60 Chinese patients with GBM, which confirmed the existence of CD95L in Chinese GBM patients and demonstrated a high degree of consistency of CD95L expression between geographically diverse Chinese and Western GBM patients;

(1) According to National Cancer Institute, standard of care treatment refers to treatment that is accepted by medical experts as a proper treatment for a certain type of disease and that is widely used by healthcare professionals. It is usually included and defined in the expert consensus or treatment guidelines, and in other words, is the level of care widely accepted in the medical community with proved efficacy and good safety in clinical practice.

SUMMARY

- (b) From April 2016 to July 2016, we submitted the Taiwan IND application in March 2016 and received approval from the TFDA in July 2016;
- (c) From September 2016 to September 2018, we completed a Phase 1 trial of CAN008 in combination with RT and TMZ in 10 patients with newly diagnosed GBM in three hospitals in Taiwan under the authorization of the TFDA. On the back of the Phase 1 trial, we filed an IND application with the NMPA in June 2017, which was accepted in July 2017. In applying for IND, we were responsible for all regulatory interactions with the NMPA's CDE and led pre-IND consultation, protocol design, medical advisory board and IND dossier preparation;
- (d) Since April 2016, in parallel with the Phase 1 trial of CAN008 in Taiwan, we have invested significant development efforts into the CMC tech transfer of CAN008 to manufacture it in China;
- (e) Since we received IND approval from the NMPA for CAN008's Phase 2 trial in March 2018, we have continued to dedicate extensive R&D efforts into the preparation of the trial, including:
 - (i) engaging experts from over 15 hospitals in China and Europe, to evaluate and refine the clinical trial protocol design;
 - (ii) assessing 9 trial clinical sites for potential participation in the trial based on their patient flow, medical capabilities and doctors' experience;
 - (iii) holding site-initiation visits at Beijing Tiantan Hospital, Tianjin Medical University General Hospital, Huashan Hospital Fudan University, Peking Union Medical College Hospital, Tongji Medical College of Huazhong University of Science & Technology and Harbin Medical University Cancer Hospital and discussed with principal investigators on study design, safety monitoring and mitigation plan, efficacy endpoints, as well as patient screening and enrollment; and
 - (iv) engaging a reputable Chinese CRO in December 2020 in preparation of our multi-center, randomized, double-blind, and placebo-controlled phase II clinical trial, for which we will design the trials, oversee clinical processes, monitor CRO's performance, and jointly share responsibility with the CRO for protocol development and investigational product management while the CRO will be responsible for clinical monitoring, pharmacovigilance, medical monitoring and data management.

SUMMARY

- (f) We had a team of 9 members, 11 members and 17 members for the research and development of CAN008 for the year ended 2019, 2020 and the six months ended June 30, 2021, respectively, covering the full life cycle of drug development including pre-clinical research, clinical operation, regulatory affair, CMC development, quality assurance, medical assistance and project management.

For more details, see “Business – Our Portfolio – CAN008 CD95-Fc fusion protein for GBM – Our R&D Efforts since In-Licensing.”

While the Phase 2 trial previously approved by the NMPA was for a second-line trial, we submitted updated Phase 2 first-line clinical trial application for CAN008 in December 2020 based on the R&D efforts as mentioned above and have received CDE clearance in April 2021. The Phase 2 trial is a mandatory trial required by the NMPA. We will independently develop CAN008 in China, and Apogenix will only play a passive role in the development process of CAN008 in China. We have engaged an industry leading CRO for the Phase 2 clinical trial, for which we will design the trials, provide guidance to the CRO’s activities, oversee clinical processes, monitor CRO’s performance, while the CRO will be responsible for clinical monitoring, pharmacovigilance, medical monitoring and data management. We have also engaged a CMO to produce raw materials for the trial. We dosed first patient in the Phase 2 clinical trial of CAN008 for the first-line treatment of GBM patients in China in October 2021. It is designed to be multi-center, randomized, double-blind and placebo-controlled to investigate the efficacy and explore the correlation of different biomarkers with treatment outcome. The trial will compare standard-of-care (tumor removal, followed by RT plus TMZ) with placebo, to standard-of-care with CAN008.

WE MAY NOT BE ABLE TO ULTIMATELY DEVELOP AND MARKET CAN008 SUCCESSFULLY.

OUR OTHER DRUG CANDIDATES

Late Stage Drug Products and Candidates

Hunterase[®] (CAN101) targeting MPS II/Hunter syndrome

Hunterase[®] (CAN101) is an enzyme replacement therapy (ERT) being developed for the treatment of mucopolysaccharidosis type II (“MPS II”). We in-licensed Hunterase[®] (CAN101) from GC Pharma (or “GC”) in January 2019. Multiple clinical trials have been conducted to date by GC to evaluate the safety and efficacy of Hunterase[®] (CAN101), including the pivotal Phase 1/2 trial for the approval from MFDS (formerly known as the Korea Food & Drug Administration or KFDA) and certain post-approval studies. The pivotal study indicated significant subject benefit, a significant decrease in uGAG level from baseline, and an improvement in the primary endpoints. Hunterase[®] (CAN101) is currently marketed in over 10 countries worldwide by GC Pharma. It received marketing authorization as an orphan drug from the MFDS in January 2012. As of the Latest Practicable Date, Hunterase[®] (CAN101) has received marketing authorization from authorities in Algeria, Belarus, Kazakhstan and Russia

SUMMARY

and has been available for prescription in Brazil, Egypt, India, Malaysia, Oman, Turkey and Venezuela, for treatment of MPS II. We obtained a clinical trial waiver on the pivotal trial and the NDA approval for Hunterase® (CAN101) for MPS II from the NMPA in September 2020. We are currently engaged in a risk screening project for CAN101 in Zhejiang Province, which has obtained the approval from the ethnics committee and Human Genetic Resources Administration of China (HGRAC), to examine the risks associated and better improve the diagnosis practice from clinical perspective. We are mainly responsible for screening patients under different tests.

We plan to conduct a post-approval study in China as required by the NMPA in the first half of 2023. For more details, please see “Business – Late Stage Drug and Drug Candidates – Hunterase® (CAN101) targeting MPS II/Hunter syndrome”.

Hunterase® (CAN101) is currently the only targeted therapy to MPS II available in China. There are eight targeted drugs for MPS II in clinical stage in China and worldwide. For details, see “Industry Overview – Mucopolysaccharidosis Type II (MPS II or Hunter syndrome)”.

CAN108 (maralixibat)

CAN108 (maralixibat) is an oral, minimally-absorbed agent designed to selectively inhibit apical sodium-dependent bile acid transporter (ASBT) and treat rare cholestatic liver diseases, including Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia (BA). In April 2021, we obtained an exclusive license from Mirum to develop, manufacture and commercialize CAN108 (maralixibat) in Greater China for ALGS, PFIC and BA. ASBT is primarily responsible for recycling bile acids from the intestine back to the liver. ASBT inhibition results 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. By targeting bile acids in these settings, maralixibat has the potential to improve long-term outcomes and symptoms in our targeted settings and provide an alternative treatment to liver transplant. Maralixibat possesses an extensive safety dataset, having been evaluated in more than 1,600 human subjects. Maralixibat has been studied in a number of completed and ongoing clinical trials in ALGS and PFIC conducted by Mirum with over 120 children treated and some on study for over seven years. Mirum obtained FDA approval for maralixibat for ALGS in September 2021.

We have started preparation of NDA for ALGS for CAN108 and expect to file a NDA in December 2021 in mainland China and Taiwan based on data obtained by Mirum, our collaboration partner, in global studies. For BA, we are supporting the patient recruitment and clinical site management in China for a Phase 2 global multi-center clinical trial initiated in May 2021 by Mirum, our collaboration partner. We target to initiate patient enrollment in China for such Phase 2 trial in the first half of 2022.

SUMMARY

Maralixibat is currently the only targeted drug for ALGS worldwide. There is currently no approved product in China or worldwide for PFIC or BA. There are one targeted drugs for ALGS, two for PFIC and four for BA being developed in China and worldwide respectively. For details, see “Industry Overview – Rare Cholestatic Liver Diseases”.

CAN106 long-acting anti-C5 antibody for complement disorders

CAN106 is a humanized monoclonal antibody against complement C5 being developed for the treatment of complement-mediated diseases including paroxysmal nocturnal hemoglobinuria (PNH), and various other complement-mediated diseases that are targeted by approved anti-C5 antibodies and other new potential indications. The molecule is originated from discovery works conducted by Privus, and Wuxi Biologics is responsible for the CMC development. We have global rights to develop and commercialize this drug candidate from both Privus and Wuxi Biologics. Based on preclinical data, CAN106 has demonstrated a favorable PK/PD profile and tolerability, indicating that CAN106 has the potential to effectively inhibit C5 in patients with PNH and potentially with reduced dosing frequency. We submitted an IND application for a Phase 1 clinical trial of CAN106 in Singapore in October 2020 and received IND approval from Health Sciences Authority (HSA) in December 2020. We initiated a Phase 1 clinical trial in healthy volunteers for CAN106 in Singapore in February 2021. This first-in-human study is designed to be a randomized, double-blind, placebo-controlled and single ascending dose study in 31 healthy volunteers to evaluate the safety, pharmacokinetics, pharmacodynamics and development of anti-drug antibodies of CAN106. A Phase 1 study is planned in China and the data readout is expected to be obtained in the first quarter of 2023. We obtained the IND approval from the NMPA for PNH in July 2021 for the Phase 1 study.

SOLIRIS is currently the only approved product in China targeting PNH. There are eight C5 inhibitors targeting PNH being developed in China and worldwide. For details, see “Industry Overview – Complement Mediated Diseases”.

We initiated a Phase 1 clinical trial in healthy volunteers for CAN106 in Singapore in February 2021, and obtained the IND approval from the NMPA for CAN106 for PNH in July 2021 for a Phase 1 study⁽¹⁾.

CAN103

CAN103 is an ERT for Gaucher disease (GD) originated from discovery works conducted by Wuxi Biologics and currently being locally developed in China by us. It is the first rare disease asset we acquired in 2018 from WuXi Biologics, which we have global proprietary rights to develop and commercialize. It is produced in an engineered cell line that produces recombinant beta-glucocerebrosidase (GCase) with highly exposed mannose for efficient uptake by macrophage and Kupfer cells in Gaucher patients to break down glucocerebrosides

(1) The IND approval from the NMPA for CAN106 for PNH is not based on the Phase I clinical data from Singapore.

SUMMARY

(GL1), the lipids that accumulate in the body of patients with GD. GD is a lysosomal storage disorder due to mutations in the GBA gene. It is one of the best known rare diseases evidenced by a large number of research literatures and has more approved drugs available as compared to other rare diseases, according to Frost & Sullivan. There were approximately 3,000 GD patients in 2020 in China.

We obtained the IND approval for CAN103 from the NMPA in October 2021. We are in preparation of a Phase 1 trial in adult and adolescent GD patients and plan to initiate patient enrollment in the first half of 2022.

There are currently six targeted drugs for GD marketed in the U.S. and seven being developed in China and worldwide. For details, see “Industry Overview – Gaucher Disease (GD)”.

Our Preclinical Candidates

CAN107

CAN107 is recombinant humanized anti-FGF23 mAb for X-linked hypophosphatemia (XLH) being developed in China currently at CMC stage in preparation to initiate IND-enabling studies. XLH is an inherited disease of phosphate metabolism where mutations inactivating the Phosphate Regulating Endopeptidase Homolog, X-Linked (PHEX) gene lead to inactivation of the PHEX protein. The lack of PHEX protein/activity prevents it from correctly regulating fibroblast growth factor 23 (FGF23), resulting in overactivity of FGF-23 that reduces vitamin D1 α -hydroxylation and phosphate reabsorption by the kidneys, leading to hypophosphatemia and the related features of hereditary hypophosphatemic rickets, local and systemic effects including impaired growth, rickets, bone abnormalities and muscular dysfunction. The prevalence of the disease is estimated at 1 in 20,000 people in 2020 and the prevalence of such inherited genetic disorder remains relatively stable over time, according to Frost & Sullivan.

CAN104

CAN104 is an ERT being developed in China for the treatment of Fabry disease (FD). FD is an inherited lysosomal storage disorder of glycosphingolipid metabolism due to the absence or deficiency of β -Gal A which can lead to life-threatening heart and kidney problems. CAN104 is a recombinant humanized β -galactosidase A enzyme (β -Gal A). Its mechanism of action is through internalization of cells by first binding to the mannose-6-phosphate receptors (M6PRs) and then transporting to the lysosome. In the lysosome, CAN104 catalyses the hydrolysis of globotriaosylceramide (GL-3) and other β -galactyl-terminated neutral glycosphingolipids. We are accelerating CAN104's preclinical development and it is currently under cell line development for IND-enabling studies. FD is one of the most common LSDs which usually starts in childhood and is more common in men than women. China has a relatively large number of patients with FD, accounting for about one-fifth of patients in the world.

SUMMARY

CAN105

CAN105 is being developed for the treatment of Hemophilia A with massive market potential. It is a recombinant, humanized, bispecific antibody that bridges activated factor IX and factor X to restore the function of the missing activated factor VIII, which is not expected to be affected by existing factor VIII inhibitors or induce new development of such inhibitors. There were over 120,000 Hemophilia A patients in China in 2020 with an expected growth at a CAGR of 0.5% from 2020 to 2025 and 0.1% from 2025 to 2030. CAN105 is expected to enter preclinical research phase in the first half of 2022.

Gene Therapy – CAN201 and CAN202

sL65 is a next generation liver-tropic AAV capsid platform for use in gene editing and gene therapy. At the American Society of Gene & Cell Therapy (“ASGCT”) conference in May 2020, data was presented showing that the capsids delivered highly efficient functional transduction of human hepatocytes in a humanized mouse model and non-human primates. The data also showed the capsids exhibited improved manufacturability and more resistance to pre-existing neutralizing antibodies in human serum samples. We are devising preclinical strategies on CAN201 as we and our collaboration partner, LogicBio, conduct preclinical evaluations of this drug candidate. Our development plan on CAN202 is subject to the development status of CAN201 to de-risk the process.

OUR PLATFORM

We are led by a management team with significant industry experience in rare diseases, spanning R&D, clinical development, regulatory affairs, business development and commercialization, supported by a pool of talent of 173 employees where 22 had a Ph.D. and/or M.D. degree and more than 80% of our employees had experience working at multinational biopharmaceutical companies as of the Latest Practicable Date. Our management team collectively has a track record of successfully commercializing rare disease therapies across the key markets including China, the United States, Europe, Latin America, and Southeast Asia. Leveraging our management’s expertise, we play an active role in advancing the rare disease industry and shaping the rare disease ecosystem in China. For example, our founder Dr. Xue is currently serving as the Deputy Director General of China’s Alliance for Rare Disease (CHARD).

Since our inception in 2012, we have built a comprehensive portfolio targeting diseases with validated mechanisms of action with significant market potential, consisting of biologics, small molecules and gene therapy solutions. We adopted an in-licensing business model and apart from our internal efforts in developing gene therapy solutions for neuromuscular disorders, all of our product pipeline as of the Latest Practical Date have been in-licensed from our business partners. We will continue to enrich it via, business partnerships and collaborations with academic institutions together with in-house research and development.

SUMMARY

- In the rare disease area, we have seven biologics and small molecules products and product candidates for the treatment of Hunter Syndrome (MPS II) and other lysosomal storage disorders (LSDs), complement mediated disorders, hemophilia A, metabolic disorders, and rare cholestatic liver diseases including Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia (BA). Among these, we obtained the marketing approval for Hunterase[®] (CAN101) for MPS II in mainland China in September 2020.
- In the rare oncology area, we are developing CAN008 for the treatment of glioblastoma multiforme (GBM). In 2018, we completed a Phase 1 clinical trial for CAN008 in Taiwan, which has successfully bridged CAN008 to Asian patients with newly diagnosed GBM on the back of clinical data previously obtained in overseas trials. We have obtained IND approval from the NMPA to commence first-line Phase 2 clinical trial of CAN008 and dosed the first patient in a Phase 2 clinical trial of CAN008 for the first-line treatment of GBM patients in mainland China in October 2021. We also obtained marketing approval for two other oncology products, Caphosol[™] (CAN002) in mainland China and Nerlynx[®] (CAN030) in Greater China.

In addition to biologics and small molecules, we are investing in next-generation technology for gene therapies. Gene therapies provide a potentially one-time, durable treatment for various rare genetic diseases. As of the Latest Practicable Date, we are using AAV sL65 capsid vector licensed in from LogicBio Therapeutics to develop two gene therapy products for the treatment of Fabry disease and Pompe disease, with options to develop two additional indications using the same vector and a clinical-stage gene editing program for the treatment of methylmalonic acidemia (MMA) pursuant to our collaboration agreements with LogicBio Therapeutics. We are also working with our research partner UMass on sponsored research programs to develop gene therapy solutions for neuromuscular disorders, with the exclusive option to license-in the assets for development. In addition, we are internally developing an adeno-associated virus (AAV) delivery platform targeting different tissues such as the central nervous system (CNS) and muscle.

COMPETITION

The pharmaceutical and biopharmaceutical industries are characterized by rapidly advancing technologies, competition and a strong emphasis on proprietary drugs. While we believe that our development experience and scientific knowledge provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biopharmaceutical companies, academic institutions and public and private research institutions. Any drug candidates that we successfully develop and commercialize will compete with existing drugs and new drugs that may become available in the future.

SUMMARY

GBM, the indication for CAN008, currently has targeted therapy treatment options for GBM in China including surgery, radiotherapy combined with TMZ concurrent chemotherapy, tumor treating field (TTF), bevacizumab (Avastin) and a bevacizumab biosimilar. The incidence of GBM in China has reached 54.7 thousand in 2020 with a CAGR of 2.0% from 2016 to 2020, and is expected to grow steadily to 59.8 thousand in 2025 and 64.4 thousand in 2030 at a CAGR of 1.8% and 1.5%, respectively. According to Frost & Sullivan, the incidence represents the total addressable market size of CAN008 for GBM. See “Industry Overview – Rare Oncology – Glioblastoma Multiforme (GBM) – Market Overview” for more details. The epidemiology of GBM was estimated by Frost & Sullivan based on the incidence rate reported by literatures and interviews from relevant experts. The expected increase in the incidence of GBM in China is primarily due to the aging of population, ionizing radiation and air pollution in the future ten years. There are currently three drugs targeting GBM marketed in China, and 22 targeted drugs for GBM being developed in China and worldwide. Our CAN008 fusion protein has competitive advantages in the treatment of GBM over other treatment options, including a validated novel mechanism of action, improved efficacy and the potential for combination therapy. For more details, see “Industry Overview – Rare Oncology – Glioblastoma Multiforme (GBM).”

MPS II, the indication for Hunterase[®] (CAN101), is also known as Hunter syndrome, which is an X-linked recessive lysosomal storage disorder. The prevalence of MPS II in Greater China reached 8,005 in 2020 and is estimated to reach 8,175 in 2030. According to Frost & Sullivan, the prevalence of MPS II represents the total addressable market size of Hunterase[®] (CAN101). See “Industry Overview – Rare Diseases – Mucopolysaccharidosis Type II (MPS II or Hunter syndrome) – Market Overview” for more details. The epidemiology of MPS II was estimated by Frost & Sullivan based on the prevalence rate reported by literatures and interviews from relevant experts. MPS II is an inherited genetic disorder, so the expected steady increase in the prevalence of MPS II in Greater China is primarily due to the increasing overall population in the future ten years. Our Hunterase[®] (CAN101) is the only approved treatment for Hunter syndrome or MPS II in China and there are three other targeted drugs approved for MPS II globally. For more details, see “Industry Overview – Rare Diseases – Mucopolysaccharidosis Type II (MPS II or Hunter syndrome).”

For the addressable market of our other product candidates, see “Industry Overview.”

RESEARCH AND DEVELOPMENT

We believe that R&D is key to driving our therapeutics strategy and maintaining our competitiveness in the biopharmaceutical industry. We are dedicated to enhancing our rare disease drugs portfolio by leveraging our strong in-house R&D capabilities, which span from preclinical research to clinical development.

SUMMARY

Our R&D team members have extensive preclinical and clinical development experience, including a proven track record in the development of drugs for the treatment of different types of rare diseases. As of the Latest Practicable Date, we had a total of 173 employees, among whom 22 have a Ph.D. and/or M.D. degree, and more than 80% of our employees had experience working at multinational biopharmaceutical companies. Our R&D team members have an average of approximately 10 years of relevant industry experience.

To leverage the capabilities of our R&D team, we promote company-wide cross-function collaboration to identify and mitigate inherent risks early in the development of innovative therapies with massive market potential. For example, in implementing such approach, our senior R&D members serve across different functional teams, and our medical team are involved from the project inception and throughout the preclinical development of our research projects. This enables our team to be familiar with the assets at an early stage so that they can formulate development ideas early on and provide feedback to the internal R&D team.

Our R&D team is led by our founder, Chairman and CEO, Dr. Xue (Ph.D., M.B.A.), a veteran entrepreneur with over 22 years of experience in medical and pharmaceutical companies. Serving as the founding general manager of Genzyme China, he had successfully managed the launch of several life-saving drugs for the treatment of hematologic cancer and rare metabolic diseases in China, including but not limited to Thymoglobulin and Cerezyme. He is also the Deputy Director General at CHARD, Vice Chair of R&D Committee of China Pharmaceutical Innovation and Research Development Association (PhIRDA) and a member of Leadership Council of Joint Institute of Peking University Health Science Center and University of Michigan Medical School.

For the years ended December 31, 2019 and 2020 and the six months ended June 30, 2021, our R&D expenses were RMB55.4 million, RMB109.6 million and RMB274.8 million, respectively. For our Core Product CAN008, our R&D expenses were RMB11.9 million, RMB4.3 million and RMB17.9 million for the years ended December 31, 2019 and 2020 and the six months ended June 30, 2021, accounting for 21.5%, 3.9% and 6.5% of our total R&D expenses, respectively.

INTELLECTUAL PROPERTY RIGHTS

Intellectual property rights are important to the success of our business. Our success depends, in part, on our ability to obtain and maintain patent and other intellectual property and proprietary protections for commercially important technologies, inventions and know-how related to our business, defend and enforce our patents, preserve the confidentiality of our trade secrets, and operate without infringing, misappropriating or otherwise violating the valid, enforceable intellectual property rights of third parties.

As of the Latest Practicable Date, we own or otherwise have exclusive rights to 17 granted patents and 47 pending patent applications worldwide. We believe there is no material legal impediment for us to obtain the approvals for these pending patents and trademarks.

SUMMARY

The following table includes the information on our key patent rights for our Core Product:

| Product | Scope of Patent Protection | Patent Applicant/ Holder | Jurisdiction | Status | Patent Expiration* |
|---------|------------------------------------------|-----------------------------|------------------|---------|--------------------|
| CAN008 | CD95-Fc Variants | Apogenix | China, Hong Kong | Granted | 2033 |
| | Reagents and methods of detecting cancer | Apogenix | China | Granted | 2033 |
| | CD95-Fc isoforms | Apogenix | China, Hong Kong | Pending | N/A |

Our Directors confirm that, as of the Latest Practicable Date, we were not a party to any material legal or administrative proceedings in connection with intellectual property rights or otherwise, and we are not aware of any instances of infringement of any third parties’ intellectual property rights by us which could materially and adversely affect our business.

For details on the portfolio of patent applications material to our business operations, see “Business – Intellectual Property”.

COLLABORATION AND LICENSING ARRANGEMENTS

Development and License Agreement with Apogenix

On June 26, 2015, we entered into a development and license agreement with Apogenix AG (previously known as Apogenix GmbH) (“Apogenix”) as amended in December 2015, May 2021 and August 2021, respectively (the “Apogenix Agreement”) concerning our exclusive right to develop, manufacture and commercialize the compound known as APG101 (CAN008) and pharmaceutical products containing APG101 (“Apogenix Licensed Products”) in Greater China.

Pursuant to the Apogenix Agreement, Apogenix granted us an exclusive, royalty-bearing, license under specified Apogenix patent rights, materials and know-how to develop (not including any modification to the compound), manufacture and commercialize, including to market, promote, label, package, distribute, import, export, offer to sell and sell the Apogenix Licensed Products in Greater China for the treatment of patients with glioblastoma disease (the “GBM”) and/or other indications. Any sublicense to a third party by us (excluding to affiliates of us and Apogenix) requires the prior written consent of Apogenix. Pursuant to the Apogenix Agreement, should we, our affiliates or sublicensees outside the scope of the agreement commence clinical trials or commercialize any CD95 ligand inhibitor for the treatment of GBM within Greater China, then Apogenix may terminate and convert our exclusive license into a non-exclusive license and may independently exploit the Apogenix Licensed Products in Greater China, and we must grant to Apogenix a non-exclusive license to our development data.

* Patent expiration does not include any applicable patent term extensions

SUMMARY

Under the Apogenix Agreement, we are responsible for the development, manufacturing and commercialization of APG101 for the treatment of GBM in Greater China. We must use commercially reasonable efforts to develop, obtain regulatory approval for and commercialize the Apogenix Licensed Products in Greater China, and we are responsible for all costs and expenses incurred by us, or by Apogenix under the development plan and technology transfer⁽¹⁾ as specified in the Apogenix Agreement associated with such activities. We will independently develop CAN008 in China, and Apogenix will only play a passive role in the development process of CAN008 in China.

For more details, see “Business – Collaboration and Licensing Arrangements – Development and License Agreement with Apogenix.”

Exclusive License Agreement with GC Pharma

On January 3, 2019, we entered into a license agreement (the “GC Pharma Agreement”) with Green Cross Corporation (“GC Pharma”) concerning the exclusive right to develop and commercialize⁽²⁾ any biopharmaceutical products containing the compound Idursulfase- β developed by GC Pharma as an active pharmaceutical ingredient that is formulated for intravenous administration in the treatment of Mucopolysaccharidosis Type II (also known as Hunter Syndrome) (the “GC Pharma Licensed Products”) in all indications except for the indication specifically for CNS symptoms (the “GC Pharma Licensed Field”). The GC Pharma Licensed Products represent the product currently marketed by or on behalf of GC Pharma outside Greater China under the product name Hunterase[®] (CAN101).

Pursuant to the GC Pharma Agreement, GC Pharma granted to us an exclusive, sublicensable (subject to certain conditions), royalty-bearing right and license under certain patent rights, know-how and product names and trademarks relating to the GC Pharma Licensed Products to develop and commercialize (excluding manufacturing activities) the GC Pharma Licensed Products in the GC Pharma Licensed Field in Greater China. We elected under the GC Pharma Agreement not to commercialize in the following provinces: Shanxi, Guizhou, Gansu, Qinghai, Xizang, Jilin and Yunnan⁽³⁾, and are required, upon written request of GC Pharma, to enter into a sublicense agreement licensing our commercialization rights under the GC Pharma Agreement to a designated GC Pharma affiliate in one or more of such provinces. GC Pharma has granted to us a right of first negotiation with respect to collaborations in the licensed territory regarding development and commercialization of the GC Pharma Licensed Products for treatment of Mucopolysaccharidosis Type II in the CNS

- (1) Technology transfer under the Apogenix Agreement mainly involved transfer of all materials relevant for the CMC process (including monochlorobimane and assay components) and information on assays. Such technology transfer was completed in 2018 according to the terms and payment schedule under the Apogenix Agreement.
- (2) GC Pharma is responsible for the manufacturing of CAN101.
- (3) We believe such provinces are less commercially attractive with lower patient affordability and health awareness. The GC Pharma Agreement does not preclude us to commercialize in these provinces if the market becomes more mature.

SUMMARY

indication. GC Pharma has also granted to us a right of first refusal with respect to GC Pharma granting to, or obtaining an offer from, a third party to develop or commercialize the GC Pharma Licensed Products in the licensed territory for treatment of Mucopolysaccharidosis Type II in the CNS indication.

Under the GC Pharma Agreement, we are responsible, and must use commercially reasonable efforts, to develop, obtain regulatory approval for and commercialize the GC Pharma Licensed Products, and we are responsible for all costs and expenses incurred by us or on behalf of us associated with such activities. Unless separately agreed, we agree to purchase and GC Pharma agrees to supply to us GC Pharma Licensed Products at a fixed price as set forth in the GC Pharma Agreement and supply samples to us for regulatory approval at no charge. We also agreed not to directly or indirectly develop, manufacture or commercialize any product indicated for the treatment of Mucopolysaccharidosis Type II in China, other than the GC Pharma Licensed Products.

For more details, see “Business – Collaboration and Licensing Arrangements – Exclusive License Agreement with GC Pharma.”

Exclusive License Agreement with WuXi Biologics

On January 7, 2019, we entered into a license agreement (the “WuXi Biologics Agreement”) with WuXi Biologics Ireland Limited (“WuXi Biologics”), wherein WuXi Biologics granted to us (i) an exclusive, transferable (subject to certain conditions), royalty-bearing license under (a) all patents and patent applications controlled by WuXi Biologics or its affiliates during the term of agreement in Greater China that claim any aspect of the anti-C5 antibody that binds specifically to the C5 protein or a pharmaceutical composition containing the anti-C5 antibody (the “WuXi Biologics Licensed Product”, representing CAN106) and (b) know-how solely pertaining to the WuXi Biologics Licensed Product, and (ii) a non-exclusive, royalty-bearing license under certain know-how that relates to both the WuXi Biologics Licensed Product and other products, in each case of (i) and (ii), with the right to sublicense through multiple tiers (subject to certain conditions), and to make, have made, use, register, sell, offer to sell, have sold, import, export, exploit, research, improve, develop and commercialize the WuXi Biologics Licensed Product (including all improvements and/or modifications) in Greater China for all indications related to the anti-C5 antibody. We granted back to WuXi Biologics a co-exclusive, irrevocable, fully paid, royalty-free license under all patent rights and know-how controlled by us, our affiliates or sublicensees at any time during the term of the agreement that is solely related to the WuXi Biologics Licensed Product or anti-C5 antibody or the research, development, manufacture, commercialization, sale or use thereof. WuXi Biologics has granted to us a right of first negotiation with respect to a global license for the WuXi Biologics Licensed Product and a right of first refusal with respect to a third party granting to or receiving from WuXi Biologics a global license, in each case outside of Greater China.

SUMMARY

Under the WuXi Biologics Agreement, we will be responsible for the development and commercialization of the WuXi Biologics Licensed Product. We must use commercially reasonable efforts to develop, obtain regulatory approval and commercialize the WuXi Biologics Licensed Product, and we are responsible for all costs and expenses incurred by us or on behalf of us associated with such activities. Unless separately agreed, WuXi Biologics is our exclusive clinical supplier and primary commercial supplier for the WuXi Biologics Licensed Product.

For more details, see “Business – Collaboration and Licensing Arrangements – Exclusive License Agreement with WuXi Biologics.”

Exclusive License Agreement with Privus

On May 9, 2020, we entered into a license agreement (the “Privus Agreement”) with Privus Biologics, LLC (“Privus”), wherein Privus granted to us (i) an exclusive, transferable (subject to certain conditions), royalty-bearing license under (a) all patents and patent applications controlled by Privus or its affiliates during the term of agreement in worldwide except for Greater China with regard to a terminal complement inhibitor of the C5a and C5b proteins, and all other terminal complement inhibitors of the C5a and C5b proteins controlled by Privus (the “Privus Licensed Product”) and (b) know-how solely pertaining to the Privus Licensed Product, and the right to sublicense through multiple tiers in worldwide except for Greater China for all Privus Licensed Product.

Under the Privus Agreement, we will have sole control over and responsibility and decision-making authority for, at our sole cost and expenses, all development and commercialization of the Privus Licensed Product. We must use commercially reasonable efforts to develop, seek regulatory approval and commercialize the Privus Licensed Product, and we are responsible for all costs and expenses incurred by us or on behalf of us associated with such activities.

For more details, see “Business – Collaboration and Licensing Arrangements – Exclusive License Agreement with Privus.”

Collaboration with UMass

On June 1, 2020, we entered into a sponsored research agreement with The University of Massachusetts as represented by and solely on behalf of its medical school (“UMass”), pursuant to which we were granted an exclusive option to obtain a worldwide, royalty-bearing, exclusive license (with the right to sublicense through multiple tiers) under certain of UMass’ patent rights and future patent rights related thereto, to use and practice such rights for the prevention, treatment, cure or control of conditions relating to certain neuromuscular disorders. Our exercise of the exclusive option obligates us and UMass to negotiate in good faith a license agreement containing reasonable terms. In consideration of the performance of research projects and the rights granted, we are obligated to provide financial support to UMass in periodic advance biannual payments as set forth in the sponsored research agreement. Pursuant

SUMMARY

to the sponsored research agreement, UMass granted to us a royalty-free, fully paid-up, perpetual, non-exclusive, worldwide license, without the right to grant sublicenses, under all of UMass' patent rights arising from the sponsored research project to make, have made, use, lease, sell, have sold, offer for sale and import products and otherwise practice such patent rights, provided that we agree to (a) demonstrate reasonable efforts to commercialize such products in the public interest and (b) pay a pro rata portion (in equal portions with each other non-exclusive licensee) of patent prosecution and maintenance costs in all countries, including the United States, in which we are granted a non-exclusive license right. Unless terminated earlier, the sponsored research agreement remains in effect until the later of two years from June 1, 2020 or completion of the research project. We may terminate the sponsored research agreement upon 30 days' prior notice if (i) a principal investigator leave UMass and no acceptable substitute is identified within 60 days or otherwise terminated his or her involvement in the research project, or (ii) parties are unable to reach agreement on the update of the research project pursuant to relevant provisions of the sponsored research agreement. Either party may terminate the sponsored research agreement upon the material uncured breach of the agreement by the other party.

On September 1, 2020, we entered into another sponsored research agreement with UMass, as represented by and solely on behalf of its medical school, for a research project on engineering AAV capsids with lower sensitivity to antibody neutralization and enhanced CNS and muscle tropism, pursuant to which we were granted an exclusive option to obtain a worldwide, royalty-bearing, exclusive license (with the right to sublicense through multiple tiers) under certain of UMass' patent rights and future patent rights related to the sponsored research project, to use and practice such rights for the prevention, treatment, cure or control of human indications, disease, disorder or conditions. Our exercise of the exclusive option obligates us and UMass to negotiate in good faith a license agreement containing reasonable terms. In consideration of the performance of research projects and the rights granted, we are obligated to provide financial support (including, material, reagents, consumables, supply and personnel costs) to UMass in periodic advance biannual payments as set forth in the sponsored research agreement. In turn, we and UMass have joint, undivided ownership of all patent rights which is conceived or reduced to practice jointly by us and UMass. Unless terminated earlier, the sponsored research agreement remains in effect until the later of two years from June 1, 2020 or completion of the research project. We may terminate the sponsored research agreement upon 30 days' prior notice if (i) a principal investigator leave UMass without acceptable substitute is identified within 60 days or otherwise terminated his or her involvement in the research project, or (ii) parties are unable to reach agreement on the update of the research project pursuant to relevant provisions of the sponsored research agreement. Either party may terminate the sponsored research agreement upon the material uncured breach of the agreement by the other party.

For more details, see "Business – Collaboration and Licensing Arrangements – Collaboration with UMass."

SUMMARY

Collaboration with LogicBio

On April 26, 2021, we entered into a strategic collaboration and licensing agreement with LogicBio Therapeutics, Inc. (“LogicBio”), wherein LogicBio granted to us (i) a worldwide, royalty-bearing, sublicensable through multiple tiers (subject to certain conditions), exclusive license to certain LogicBio patents and know-how to develop, manufacture and commercialize gene therapy candidates for two targets for the treatment of Fabry and Pompe diseases, such LogicBio patents and know-how being inclusive of LogicBio’s adeno-associated virus (AAV) sL65, a capsid produced from the LogicBio sAAVy™ platform; (ii) options for the development of AAV sL65-based treatments for two additional targets; and (iii) an option to obtain an exclusive, royalty-bearing, sublicensable through multiple tiers (subject to certain conditions) license to LogicBio patents and know-how to LB-001, an investigational in-vivo gene editing technology based on GeneRide™ platform for the potential treatment of methylmalonic acidemia (MMA), in Greater China (collectively, the “LogicBio Licensed Products”). Pursuant to the agreement, we granted to LogicBio a royalty-free, non-exclusive, sublicensable, license for LogicBio to perform its obligations under the agreement.

We are required to use commercially reasonable efforts to develop and seek regulatory approval for LogicBio Licensed Product directed against each target corresponding to each licensed indication in certain countries, and upon approval of the applicable biologics license application in such country, we are obligated to use commercially reasonable efforts to obtain regulatory approval and commercialize such product in such country. Similarly, if we exercise the LB-001 option, we are required to use commercially reasonable efforts in Greater China to develop, seek regulatory approval and commercialize LogicBio Licensed Product for LB-001. Except as otherwise provided in the agreement, we are solely responsible for, and will have sole control over, preparing, filing, and maintaining regulatory submissions and communicating with regulatory authorities in Greater China with respect to LogicBio Licensed Products.

Subject to the terms of the agreement, LogicBio will have an option, on a target-by-target basis with respect to certain targets, to enter into a separate worldwide, co-exclusive (with us) co-development and co-commercialization agreement with us with regard to products that are directed to the applicable target in certain time period. We and LogicBio will have sole responsibility for the conduct of the activities allocated to us or LogicBio, respectively.

Subject to the terms of the agreement, on a product-by-product basis for products other than LB-001, during an initial LogicBio manufacturing period, LogicBio has sole responsibility for all manufacturing activities. Following the initial LogicBio manufacturing period, we will have sole responsibility for and sole decision-making authority with respect to all manufacturing activities.

For more details, see “Business – Collaboration and Licensing Arrangements – Collaboration with LogicBio.”

SUMMARY

Collaboration with Mirum

On April 28, 2021, we entered into a license agreement with Mirum Pharmaceuticals, Inc. (“Mirum”), wherein Mirum granted to us an exclusive, royalty-bearing, sublicensable (subject to certain conditions) license to certain Mirum licensed know-how and patents to develop, manufacture and commercialize maralixibat, an investigational, orally administered medication, and pharmaceutical products containing maralixibat (“Mirum Licensed Products”), which is being evaluated in several indications including Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia (BA), within the licensed territory of Greater China for ALGS, PFIC, and BA. The licenses granted to us constitute sublicenses of upstream license agreements to Mirum which Mirum may not amend or terminate without our prior written consent.

In collaboration with Mirum, we have agreed to oversee Mirum’s clinical study sites in China, with the goal of accelerating enrollment of the global Phase 2b EMBARK study, which was recently initiated for patients with BA. We also have the right to manufacture maralixibat in Greater China under certain conditions. We are required to use commercially reasonable efforts, at our sole cost and expense, to develop and commercialize the Mirum Licensed Products in Greater China and are responsible for obtaining regulatory approval for Mirum Licensed Products in the licensed territory.

For more details, see “Business – Collaboration and Licensing Arrangements – Collaboration with Mirum.”

SALES AND MARKETING

As of the Latest Practicable Date, we had commercialized three products, Caphsol™ (CAN002) in mainland China, Nerlynx® (CAN030) in Greater China, and Hunterase® (CAN101) in mainland China. We use a combination of our in-house sales and marketing team and a network of independent distributors to sell our products in Greater China. Our management team has a track record of successfully commercializing rare disease therapies across various key markets including China, Southeast Asia, the United States, Latin America and Europe. Led by them, we have assembled an experienced commercial team to ensure the successful commercialization of our drug candidates upon approval. As of the Latest Practicable Date, we had established a commercial team jointly led by Mr. Yijun Lu, our China General Manager and Mr. Marcelo Cheresky, our Chief Business Officer, consisting of 81 members. We expect to expand our commercialization team to over 300 members in the next five years, comprising of three major functions including marketing and sales, medical affairs and patient advocacy and assistance, with the mission to execute medical engagement plan for KOL development, promote community awareness and explore industry insights for better drug development strategies.

SUMMARY

We employ a strategic marketing model to increase our market penetration and to promote our products. Under this model, we promote our products to hospitals and physicians in Greater China through academic marketing, establishing center of excellence and referral network, and providing trainings to physicians. In particular, a series of marketing activities have been carried out for our close-to-launch product candidates such as CAN108, including establishing collaboration with hospitals with expertise in our targeted rare diseases, KOL engagement through regional educational seminars held online and offline with the Primary Health Care Foundation of China. We plan to follow similar marketing strategies as CAN008 and other pipeline products approach commercialization stage. With more late-stage drug candidates entering into the commercialization stage, we also plan to set up local offices in each key province across the country. See “Our Strategies – Drive commercialization of our late-stage assets in Greater China.”

Pricing

When determining the price of our products, we consider factors such as clinical value, current unmet medical needs, product quality, production costs, health economics in the country to market in, patient affordability and competitors’ pricing strategies. The list price of Hunterase[®] (CAN101) in China is similar to its reimbursement price in South Korea, where it was previously approved and commercialized taking the afore-mentioned key factors into account. We have initiated a Hunterase[®] patient assistance program in collaboration with a medical payment services provider to improve patients’ access to Hunterase[®] (CAN101) in China. We expect to follow similar pricing strategies when CAN008 enters the commercialization stage. We believe our pricing strategy can strike a balance between the affordability of patients and a sustainable return on these products.

As of the Latest Practicable Date, CAN008, our Core Product was still at clinical stage, and our three commercialized products, CAN101, CAN002 and CAN030, had not been included under the national or provincial public medical insurance program in China or other relevant regions. In light of the current circumstances, we believe that the likelihood that our products will be included in the national public medical insurance program in China in the near future remains relatively low. However, we observed that over the years of China’s exploration in insurance mechanism of rare diseases at the local level, an aggregate of 29 provinces have implemented insurance policies for certain rare disease with various reimbursement models. If our commercialized products or Core Product upon commercialization are included in public medical insurance programs, we may face downward pricing pressure. Nevertheless, it will also increase the sales volume and therefore further promote the market growth of our products.

CUSTOMERS

For the years ended December 31, 2019 and 2020 and the six months ended June 30, 2021, the aggregate sales to our five largest customers were RMB1.5 million, RMB9.4 million and RMB6.4 million, representing 100.0%, 77.7% and 52.4% of our revenue, respectively. Sales to our largest customer for the same periods were RMB1.1 million, RMB5.3 million and RMB2.2 million, representing 72.2%, 44.2% and 17.7% of our revenue, respectively.

SUMMARY

RAW MATERIALS AND SUPPLIERS

During the Track Record Period, our suppliers primarily consisted of (i) licensors from which we obtained intellectual property rights in respect of our in-licensed drug candidates and (ii) CROs and CDMOs, who provide third-party contracting services for research and development. We select our suppliers by considering their product quality, industry reputation and compliance with relevant regulations and industry standards.

For the years ended December 31, 2019 and 2020 and the six months ended June 30, 2021, purchases from our five largest suppliers in aggregate accounted for 63.1%, 83.7% and 69.3% of our total purchases (including value added tax), respectively, and purchases from our largest supplier accounted for 42.8%, 55.6% and 23.3% of our total purchases for the same periods (including value added tax), respectively. During the Track Record Period, our purchases mainly include rights under license agreement and R&D services.

During the Track Record Period, we have not procured raw materials or equipment for commercial manufacturing.

OUR COMPETITIVE STRENGTHS

We believe the following strengths differentiate us from our competitors:

- A rare disease focused biopharmaceutical company dedicated to addressing unmet medical needs
- A comprehensive portfolio of rare disease focused therapies with significant revenue potential
- Extensive strategic partnerships to source innovative therapies globally
- A rare disease platform positioned to drive rapid and comprehensive product development and market access in China and globally
- Management team with deep industry experience and a track record of commercializing rare disease therapies globally

OUR STRATEGIES

We aspire to become a global biopharmaceutical leader that delivers targeted therapies for rare disease patients in China and worldwide. We plan to implement the following strategies to achieve our vision:

- Further solidify our position in the China’s rare disease ecosystem and build a global rare diseases franchise
- Drive commercialization of our late-stage assets in Greater China

SUMMARY

- Rapidly advance and expand our portfolio
- Maximize value creation through partnership and collaboration
- Enhance capabilities with in-house drug research, development and manufacturing infrastructure in global and Greater China markets

OUR SINGLE LARGEST SHAREHOLDER

The Company had no controlling shareholder as defined under the Listing Rules as at the Latest Practicable Date. As of the Latest Practicable Date, Dr. Xue, our founder, Chairman of the Board, executive Director and CEO, is entitled to ultimately control an aggregate of 18.84% of the voting rights of the Company, being more voting rights than any other Shareholder of the Company, through (i) Dr. Xue (holding beneficially under his own name), (ii) CTX Pharma (an investment holding entity wholly-owned by Dr. Xue), (iii) the Voting Rights Proxy Agreement and (iv) the Family Trust. The Voting Rights Proxy Agreement shall terminate upon [REDACTED]. Immediately following the completion of the Share Subdivision and the [REDACTED] (assuming that the [REDACTED] is not exercised and without taking into account any additional Shares to be issued upon the exercise of the Share Options granted under the [REDACTED] Equity Incentive Plan), Dr. Xue is expected to ultimately control an aggregate of [REDACTED]% of the voting rights of the Company, which continues to be more than any other Shareholders, through (i) himself beneficially, (ii) CTX Pharma and (iii) the Family Trust. See sections headed “History, Reorganization and Corporate Structure – Our Structure Immediately Prior to the Share Subdivision, Conversion and [REDACTED]”, “History, Reorganization and Corporate Structure – Our Structure Immediately Following the [REDACTED]” and “Substantial Shareholders” for further details.

OUR [REDACTED] INVESTORS

Since 2015, we have secured [REDACTED] investments of an aggregate amount of approximately US\$269 million pursuant to the respective investment agreements. Our [REDACTED] Investors includes certain Sophisticated Investors, such as WuXi PharmaTech Healthcare Fund I L.P., WuXi AppTec (HongKong) Limited, RA Capital Health Fund, L.P., RA Capital Nexus Fund L.P., Blackwell Partners LLC – Series A, Qiming Venture Partners IV, L.P. and Qiming Managing Directors Fund IV, L.P. Please refer to the section headed “History, Reorganization and Corporate Structure – [REDACTED] Investments” in this document.

SUMMARY OF KEY FINANCIAL POSITIONS

This summary historical data of financial information set forth below have been derived from, and should be read in conjunction with, our consolidated financial statements, including the accompanying notes, set forth in the Accountants’ Report set out in Appendix I to this document, as well as the information set forth in “Financial Information” of this document. Our financial information was prepared in accordance with IFRSs.

SUMMARY

Summary Data from Consolidated Statements of Profit or Loss

The table below sets forth our consolidated statements of profit or loss for the periods indicated derived from our consolidated statements of profit or loss set out in the Accountants’ Report included in Appendix I to this document:

| | For the year ended | | For the six months | |
|---------------------------------------------------------------|--------------------|-----------|--------------------|-----------|
| | December 31, | | ended June 30, | |
| | 2019 | 2020 | 2020 | 2021 |
| | RMB’000 | RMB’000 | RMB’000 | RMB’000 |
| | | | <i>(unaudited)</i> | |
| Revenue | 1,469 | 12,032 | 1,944 | 12,192 |
| Cost of sales | (504) | (5,154) | (838) | (5,353) |
| Gross profit | 965 | 6,878 | 1,106 | 6,839 |
| Other income and gains | 580 | 1,359 | 747 | 11,052 |
| Selling and distribution expenses | (28,881) | (51,008) | (16,401) | (44,768) |
| Administrative expenses | (53,719) | (77,716) | (29,337) | (52,928) |
| Research and development expenses | (55,383) | (109,642) | (35,884) | (274,837) |
| Fair value changes of convertible redeemable preferred shares | (73,694) | (591,385) | (79,043) | (21,848) |
| Fair value changes of convertible loans | (1,584) | 1,689 | 1,689 | – |
| Fair value changes of derivative financial instruments | (17) | (20,746) | 3,175 | 34,454 |
| Other expenses | (3,667) | (1,599) | (663) | (609) |
| Finance costs | (2,275) | (3,873) | (2,119) | (1,558) |
| Loss before tax | (217,675) | (846,043) | (156,730) | (344,203) |
| Income tax expense | – | – | – | – |
| Loss for the year/period | (217,675) | (846,043) | (156,730) | (344,203) |
| Attributable to: | | | | |
| Owners of the parent | (217,675) | (846,043) | (156,730) | (344,203) |

During the Track Record Period, our revenue was generated from sales of medical products, including our CaphosolTM(CAN002), Nerlynx[®] (CAN030) and Hunterase[®] (CAN101) to three countries or regions. The increase in revenue over the Track Record Period is primarily attributable to the increase in sales of our Nerlynx[®] (CAN030) since its launch in Hong Kong in December 2019, in mainland in China in November 2020 and in Taiwan in December 2020. For the year ended 2019 and 2020 and the six months ended June 30, 2021, the revenue we generated from CaphosolTM(CAN002) was RMB1.1 million, RMB0.4 million and RMB0.9 million, respectively, and the revenue we generated from Nerlynx[®] (CAN030) was RMB0.4 million, RMB11.7 million and RMB10.7 million, respectively. For the six months ended June 30, 2021, the revenue we generated from Hunterase[®] (CAN101) was RMB0.7 million.

SUMMARY

Geographical information

| | Year ended December 31, | | | | Six months ended June 30, | | | |
|-----------|-------------------------|--------------------|---------------|--------------------|---------------------------|--------------------|---------------|--------------------|
| | 2019 | | 2020 | | 2020 | | 2021 | |
| | RMB'000 | % of total revenue | RMB'000 | % of total revenue | RMB'000 | % of total revenue | RMB'000 | % of total revenue |
| | | | | | <i>(unaudited)</i> | | | |
| Mainland | | | | | | | | |
| China | 1,061 | 72.2% | 5,448 | 45.3% | 117 | 6.0% | 3,837 | 31.5% |
| Taiwan | – | – | 319 | 2.6% | – | – | 5,418 | 44.4% |
| Hong Kong | 408 | 27.8% | 6,265 | 52.1% | 1,827 | 94.0% | 2,937 | 24.1% |
| | <u>1,469</u> | <u>100.0%</u> | <u>12,032</u> | <u>100.0%</u> | <u>1,944</u> | <u>100.0%</u> | <u>12,192</u> | <u>100.0%</u> |

The revenue information above is based on the locations of the customers.

The increase in our net losses in 2020 is primarily attributable to the increase in our fair value changes of convertible redeemable preferred shares from a loss of RMB73.7 million for 2019 to a loss of RMB591.4 million for 2020, primarily due to the increase in our Company’s valuation. The increase in our net losses for the six months ended June 30, 2021 is primarily attributed to (i) the increased license fee from RMB19.2 million for the six months ended June 30, 2020 to RMB173.3 million for the six months ended June 30, 2021, and (ii) increased testing and clinical trial expenses from RMB1.7 million for the six months ended June 30, 2020 to RMB67.0 million for the six months ended June 30, 2021, due to more CRO and CMC activities carried out for our pipeline candidates in the six months ended June 30, 2021 as compared with those in the same period of 2020. For more details, see “Financial Information – Description of Selected Components of Statements of Profit or Loss” in this document.

The fair value changes of convertible redeemable preferred shares adversely affected and will continue to affect our performance during and subsequent to the Track Record Period until the conversion of preferred shares into ordinary shares upon [REDACTED]. While the fair value loss of financial instruments issued to [REDACTED] investors has adversely impacted our financial position during the Track Record Period, the financial instruments will be automatically converted into Shares upon the [REDACTED], after which we do not expect to recognize any further loss or gain on fair value changes from the convertible redeemable preferred shares. The convertible redeemable preferred shares will be re-designated from financial liabilities to equity as a result of the automatic conversion of preferred shares into ordinary shares upon [REDACTED], thereby turning the net liabilities position into a net assets position. For more information, please refer to the section headed “Risk Factors – Risks Relating to Our Financial Position and Need for Additional Capital – Our results of operations, financial condition and prospects may be adversely affected by fair value changes in our convertible redeemable preferred shares, convertible loan and derivative financial instruments at fair value through profit or loss.”

SUMMARY

Summary Data from Consolidated Statements of Financial Position

The table below sets forth selected information from our consolidated statements of financial position as of the dates indicated, which have been extracted from the Accountants’ Report set out in Appendix I to this document:

| | As of December 31, | | As of |
|-----------------------------------------|--------------------|--------------------|--------------------|
| | 2019 | 2020 | June 30, 2021 |
| | <i>RMB’000</i> | <i>RMB’000</i> | <i>RMB’000</i> |
| Total non-current assets ⁽¹⁾ | 50,645 | 195,313 | 70,939 |
| Total current assets | 37,905 | 391,045 | 480,432 |
| Total assets | 88,550 | 586,358 | 551,371 |
| | | | |
| Total current liabilities | 43,749 | 108,103 | 100,925 |
| Total non-current liabilities | 1,035,447 | 2,224,111 | 2,515,244 |
| Total liabilities | 1,079,196 | 2,332,214 | 2,616,169 |
| | | | |
| Net current (liabilities)/assets | (5,844) | 282,942 | 379,507 |
| Net liabilities | (990,646) | (1,745,856) | (2,064,798) |
| | | | |
| Share capital | 5 | 5 | 5 |
| Reserves | (990,651) | (1,745,861) | (2,064,803) |
| | | | |
| Total equity | (990,646) | (1,745,856) | (2,064,798) |

We recorded net current liabilities of RMB5.8 million and net current assets of RMB282.9 million as of December 31, 2019 and December 31, 2020, respectively. As of June 30, 2021, we had net current assets of RMB379.5 million. As of [August 31], 2021, being the latest practicable date for the purpose of liquidity disclosure in this document, we had net current assets of RMB316.7 million. The Company had net current liabilities of RMB5.8 million as of December 31, 2019, which were primarily due to large amount of other payables and accruals and Interest-bearing bank and other borrowings. The Company recorded net liabilities of RMB990.6 million, RMB1,745.9 million and RMB2,064.8 million as at December 31, 2019, 2020 and June 30, 2021, respectively, mainly due to the convertible redeemable preferred shares which were issued through several rounds of financing arrangements and are measured at fair value as at December 31, 2019, 2020 and June 30, 2021 as liabilities in the consolidated statements of financial position.

For more details, see “Financial Information – Discussion of Certain Selected Items from the Consolidated Statements of Financial Position” in this document.

SUMMARY

Summary Data from Consolidated Cash Flow Statements

The following table sets forth summary data from our consolidated statements of cash flows for the periods indicated:

| | As of December 31, | | As of June 30, | |
|--------------------------------------------------------------------------|---------------------------|----------------|-----------------------|----------------|
| | 2019 | 2020 | 2020 | 2021 |
| | <i>RMB'000</i> | <i>RMB'000</i> | <i>RMB'000</i> | <i>RMB'000</i> |
| Cash flows from operating activities before movements in working capital | (116,560) | (198,611) | (67,740) | (351,521) |
| Changes in working capital | (9,735) | 45,999 | (2,902) | (10,762) |
| Interest received | 120 | 964 | 454 | 1,124 |
| Net cash flows used in operating activities | (126,175) | (151,648) | (70,188) | (361,159) |
| Net cash flows from/(used in) investing activities | (42,420) | (153,483) | (146,104) | 128,581 |
| Net cash flows from/(used in) financing activities | 96,967 | 679,263 | 397,538 | 315,383 |
| Net increase/(decrease) in cash and cash equivalents | (71,628) | 347,132 | 181,246 | 82,805 |
| Cash and cash equivalents at beginning of year | 85,240 | 13,873 | 13,873 | 360,804 |
| Effect of foreign exchange rate changes, net | 261 | (27,201) | (470) | (1,509) |
| Cash and cash equivalents at end of year | 13,873 | 360,804 | 194,649 | 442,100 |

During the Track Record Period, we incurred negative net cash flows from operations, substantially due to our research and development expenses. Our operating cash flow will continue to be affected by our research and development and selling and distribution expenses. During the Track Record Period and up to the Latest Practicable Date, we primarily funded our working capital requirements through proceeds from private equity financing. Our management closely monitors uses of cash and cash balances and has maintained a healthy liquidity for our operations and as our business develops and expands, we expect to generate more cash flow from our operating activities, through launching and commercializing our products upon approval and enhancing our operating efficiency.

Since the commencement of our business operation, we have incurred negative cash flows from our operations. Substantially all of our operating cash outflows have resulted from our R&D costs, selling and distribution expenses and administrative expenses. In view of our net operating cash outflows throughout the Track Record Period, we plan to improve such position by (i) further increase our sales of our approved products; (ii) rapidly advancing our late-stage

SUMMARY

pipeline products towards commercialization to generate revenue from product sales, (iii) adopting comprehensive measures to effectively control our cost and operating expenses, primarily including research and development costs, sales and distribution expenses and administrative expenses; (iv) enhancing working capital management efficiency; (v) successfully launching the [REDACTED] to obtain the [REDACTED]; and (vi) seeking additional funding through public or private offerings, debt financing, collaboration and licensing arrangements or other sources, if needed.

Our cash burn rate refers to the average monthly (i) net cash used in operating activities, which includes research and development expenses, and (ii) capital expenditures. We had bank balance and cash of RMB381.5 million as of [August 31], 2021. We estimate that we will receive net [REDACTED] of approximately HK\$[REDACTED] after deducting the [REDACTED] fees and expenses payable by us in the [REDACTED], assuming no [REDACTED] is exercised and assuming an [REDACTED] of HK\$[REDACTED] per [REDACTED], being the mid-point of the indicative [REDACTED] range of HK\$[REDACTED] to HK\$[REDACTED] per [REDACTED] in this Document. Assuming an average cash burn rate going forward of the same level in 2020, we estimate that our cash and cash equivalents as of [August 31], 2021 will be able to maintain our financial viability for 15 months. Assuming an average cash burn rate going forward of two times the level in 2020, we estimate that our cash and cash equivalents as of [August 31], 2021 will be able to maintain our financial viability for 36 months, if we take into account the estimated net [REDACTED] from the [REDACTED]. We will continue to monitor our working capital, cash flows, and our business development progress.

Key Financial Ratios

The table below sets forth the key financial ratios of our Group for the periods or as of the dates indicated:

| | For the year ended | | For the six months | |
|------------------------------|--------------------|-------|---------------------|-----------------|
| | December 31, | | ended June 30, | |
| | 2019 | 2020 | 2020 | 2021 |
| Gross margin ⁽¹⁾ | 65.7% | 57.2% | 56.9% | 56.1% |
| | | | As of | As of |
| | | | December 31, | June 30, |
| | | | 2019 | 2020 |
| Current ratio ⁽²⁾ | | 86.6% | 361.7% | 476.0% |

Notes:

- (1) Gross margin equals gross profit divided by revenue as of the end the year/period.
- (2) Current ratio equals current assets divided by current liabilities as of the end of the year/period.

SUMMARY

Our gross profit margin decreased from 65.7% for 2019 to 57.2% for 2020 due to adjustments in business strategy and decreases in the average selling prices of our commercialized products. Our gross profit margin stayed stable at 56.9% for the six months ended June 30, 2020 and 56.1% for the six months ended June 30, 2021. For more information on the change in our gross profit margin, see “Financial Information – Year Ended December 31, 2019 Compared to Year Ended December 31, 2020 – Gross Profit and Gross Profit Margin” and “Financial Information – Six Months Ended June 30, 2020 Compared to Six Months Ended June 30, 2021 – Gross Profit and Gross Profit Margin.”

[REDACTED]

SUMMARY

USE OF [REDACTED]

We estimate that we will receive net [REDACTED] from the [REDACTED] of approximately HK\$[REDACTED], after deducting [REDACTED] commissions, fees and estimated expenses payable by us in connection with the [REDACTED], assuming no [REDACTED] is exercised and assuming an [REDACTED] of HK\$[REDACTED] per Share, being the mid-point of the indicative [REDACTED] range stated in this document.

We intend to use the net [REDACTED] for the following purposes, subject to changes in light of our evolving business needs and changing market conditions:

- Approximately [REDACTED]%, or HK\$[REDACTED], will be allocated to fund the ongoing and future R&D (including planned clinical trials, preparation of registration filings and milestone fees), and CMC development and manufacturing of our Core Product candidate CAN008 (primarily including facilities under construction in Suzhou that will cover the process development and clinical trial materials production in GMP environment for CAN008; the clinical trial materials production can also be transferred to Suzhou facility from current CMO);
- Approximately [REDACTED]%, or HK\$[REDACTED], will be allocated to fund our major products and product candidates in our pipeline;
 - i. Approximately [REDACTED]%, or HK\$[REDACTED], is expected to fund the ongoing commercialization, post-approval study and milestone fees of Hunterase[®] (CAN101);
 - ii. Approximately [REDACTED]%, or HK\$[REDACTED], is expected to fund the ongoing and future R&D (including ongoing and planned clinical trials in Singapore and China, preparation of registration filings and milestone fees) of CAN106;
 - iii. Approximately [REDACTED]%, or HK\$[REDACTED], is expected to fund the ongoing and future R&D (including ongoing and planned clinical trials, preparation of registration filings and milestone fees) of CAN103;
 - iv. Approximately [REDACTED]%, or HK\$[REDACTED], is expected to fund the ongoing and future R&D (including ongoing and planned clinical trials, preparation of registration filings and milestone fees) and future commercial launches (including sales and marketing) of CAN108;
- Approximately [REDACTED]%, or HK\$[REDACTED], will be allocated to fund ongoing and future R&D (including ongoing and planned clinical trials, preparation of registration filings and milestone fees) of other non-gene therapy products and product candidates in our pipeline;
- Approximately [REDACTED]%, or HK\$[REDACTED], is expected to fund the ongoing and future R&D (including ongoing and planned clinical trials, preparation of registration filings and milestone fees) of CAN201, CAN202 and our other gene therapy programs;

SUMMARY

The remaining [REDACTED]% of the net [REDACTED], or HK\$[REDACTED], will be allocated to fund the R&D and other general business purposes as follows:

- Approximately [REDACTED]%, or HK\$[REDACTED], will be allocated to develop our R&D and manufacturing facilities in both China and the U.S. for all our products and drug candidates, and potential office and site expansion and upgrade in China and the U.S. The [REDACTED] allocated to the R&D and manufacturing facilities in China under this item refers to the costs associated with the facilities under construction in Suzhou that will be used to develop and manufacture our products and drug candidates other than CAN008. There is no overlap of the use of [REDACTED] for R&D and manufacturing facilities under this item and CMC development and manufacturing of CAN008;
- Approximately [REDACTED]%, or HK\$[REDACTED], will be allocated to our other R&D activities including employment costs in both China and the U.S.;
- Approximately [REDACTED]%, or HK\$[REDACTED], will be allocated for potential strategic acquisitions, investments, in-licensing or collaborations. We do not have any concrete acquisition target but plan to explore drug candidates in the rare disease and gene therapy area which may be complimentary to our current drug portfolio;
- Approximately [REDACTED]%, or HK\$[REDACTED], will be used for our commercialization activities, including expanding our sales and marketing team; and
- Approximately [REDACTED]%, or HK\$[REDACTED], will be used for our working capital and general corporate purposes.

For further details, see “Future Plans and Use of [REDACTED]”.

DIVIDEND

No dividend has been paid or declared by us during the Track Record Period. You should note that historical dividend distributions are not indicative of our future dividend distribution policy.

We are a holding company incorporated in the Cayman Islands. We may need dividends and other distributions on equity from our PRC subsidiary to satisfy our liquidity requirements. Current PRC regulations permit our PRC subsidiary to pay dividends to us only out of their accumulated after-tax profits, if any, determined in accordance with PRC accounting standards and regulations. In addition, our PRC subsidiary is required to set aside at least 10% of their respective accumulated after-tax profits each year, if any, to fund certain reserve funds until the total amount set aside reaches 50% of their respective registered capital. Our PRC subsidiary may also allocate a portion of its after-tax profits based on PRC accounting standards to

SUMMARY

employee welfare and bonus funds at their discretion. These reserves are not distributable as cash dividends. Furthermore, if our PRC subsidiary incurs debt on their own behalf in the future, the instruments governing the debt may restrict their ability to pay dividends or make other payments to us.

We currently expect to retain all future earnings for use in the operation and expansion of our business and do not anticipate paying cash dividends in the foreseeable future. Any declaration and payment as well as the amount of dividends will be subject to our constitutional documents and the Cayman Companies Act. The declaration and payment of any dividends in the future will be determined by our Board, in its discretion, and will depend on a number of factors, including our earnings, capital requirements, overall financial condition and contractual restrictions. Our Shareholders in a general meeting may approve any declaration of dividends, which must not exceed the amount recommended by our Board. As advised by our Cayman Islands legal adviser, under the Cayman Companies Act, a Cayman Islands company may pay a dividend out of either profits or share premium account, provided that in no circumstances may a dividend be paid if this would result in our Company being unable to pay its debts as they fall due in the ordinary course of business. In light of our accumulated losses as disclosed in this document, it is unlikely that we will be eligible to pay a dividend out of our profits in the foreseeable future. We may, however, pay a dividend out of our share premium account unless the payment of such a dividend would result in our Company being unable to pay our debts as they fall due in the ordinary course of business. There is no assurance that dividends of any amount will be declared to be distributed in any year.

[REDACTED] EXPENSES

The total [REDACTED] expenses (including [REDACTED] commissions) payable by our Company are estimated to be approximately HK\$[REDACTED] (or approximately US\$ [REDACTED]) constituting approximately [REDACTED]% of the gross [REDACTED] from the [REDACTED], assuming the [REDACTED] is not exercised and based on an [REDACTED] of HK\$[REDACTED] (being the mid-point of our [REDACTED] range of HK\$[REDACTED] to HK\$[REDACTED] per [REDACTED]). These [REDACTED] expenses mainly comprise legal and other professional fees paid and payable to the professional parties, commissions payable to the [REDACTED], and printing and other expenses for their services rendered in relation to the [REDACTED] and the [REDACTED].

In 2019, 2020 and for the six months ended June 30, 2021, [REDACTED] expenses charged to our consolidated statements of profit or loss were HK\$[REDACTED], HK\$[REDACTED], and HK\$[REDACTED], respectively. After June 30, 2021, approximately HK\$[REDACTED] is expected to be charged to our consolidated statements of profit or loss, and approximately HK\$[REDACTED] is expected to be charged against equity upon the [REDACTED]. The [REDACTED] expenses above are the latest practicable estimate for reference only, and the actual amount may differ from this estimate.

SUMMARY

RECENT DEVELOPMENTS

Recent Update in Clinical Development

In October 2021, we dosed the first patient in a first-line Phase 2 clinical trial for CAN008 on GBM patients in China. The trial is designed to be multi-center, randomized, double-blind and placebo-controlled to investigate the efficacy and explore the correlation of different biomarkers with treatment outcome. For details, please refer to “Business – CAN008 CD95-Fc fusion protein for GBM – Clinical Development Plan.”

We also obtained the IND approval for CAN103 from the NMPA in October 2021. We are in preparation of a Phase 1 trial in adult and adolescent GD patients and plan to initiate patient enrollment in the first half of 2022.

Newly-commercialized Product in May 2021

We obtained the marketing approval for Hunterase[®] (CAN101) for MPS II in mainland China in September 2020. We delivered the first commercial prescription of Hunterase[®] (CAN101) in China in May 2021 and the China treatment consensus that includes Hunterase[®] (CAN101) as the standard of care ERT was published in June 2021. Hunterase[®] (CAN101) was allowed to be manufactured after issuance of the Import Drug License, which we obtained after the process of manufacturing, packaging and release preparations was completed over a period of three months. In addition, Hunterase[®] (CAN101) had to pass the batch-by-batch in-country testing as required by the NMPA before commercialization, which took another three months to complete. Therefore, we were only able to deliver the first commercial prescription in May 2021 after it passed the regulatory and testing requirements in China for imported biologics. We expect to gradually commence nationwide sales of Hunterase[®] (CAN101) in Greater China.

Recent Business Development Efforts

In October 2021, we entered into a research collaboration and license agreement with Scriptr Global, Inc., which granted us exclusive worldwide rights to develop, manufacture and commercialize a gene therapy candidate for the treatment of dystrophinopathies.

In October 2021, we entered into a sponsored research agreement with the University of Washington School of Medicine, in Seattle, Washington, for gene therapy research in Duchenne muscular dystrophy (DMD), a rare neuromuscular disease.

Expected Net Loss in 2021

We expect the net loss in 2021 will substantially increase as compared with 2020, primarily due to expected increases in (i) R&D expenses in relation to both pre-clinical and clinical studies; and (ii) expenses in connection with the [REDACTED] incurred in 2021.

SUMMARY

Expected Further Change in Fair Value Change of Convertible Redeemable Preferred Shares in 2021

We expect to recognize additional loss from the fair value changes of the convertible redeemable preferred shares after June 30, 2021 to the [REDACTED]. After the automatic conversion of the convertible redeemable preferred shares into Shares upon the [REDACTED], which will result in a net asset position, we do not expect to recognize any further loss or gain on fair value changes from the convertible redeemable preferred shares in the future.

Impact of the COVID-19 Outbreak

Since the end of December 2019, the outbreak of COVID-19 has materially and adversely affected the global economy. In response, local governments has imposed widespread lockdowns, closure of work places and restrictions on mobility and travel to contain the spread of the virus. As of the Latest Practicable Date, substantially all of the Chinese cities had eased or lifted domestic travel restrictions and resumed normal social activities, work and production.

The government lockdown and other restrictive measures had resulted in significantly reduced mobility of our employees, causing most of the employees to work remotely during early phases of COVID-19 outbreak. As a result, we had implemented various precautionary measures and adjusted our employee’s work arrangements according to the relevant regulations and policies, which had allowed us to maintain a sufficient number of personnel on-site who managed to work under flexible schedule to continue our research and development activities.

There has not been any material disruption of our ongoing clinical trials and other R&D work for our product candidates since the outbreak of COVID-19 pandemic. We have not experienced and currently do not expect any material delays in regulatory affairs with respect to our clinical trials or any long-term impact on our operation or deviation from our overall development plans due to the COVID-19 pandemic. Our Directors are of the view that the COVID-19 pandemic is not expected to have any material adverse impact on the Group’s overall business operation.

To some extent, reduced transportations and disruption to manufacturing and logistics networks in China due to the COVID-19 outbreak affected our suppliers’ abilities to manufacture and transport consumables, equipment and other supplies necessary for our operations. Nevertheless, as of the Latest Practicable Date, most of our suppliers had resumed normal operations and we had not experienced any material disruption or shortage of supplies during the COVID-19 outbreak since the outbreak of COVID-19.

As of the Latest Practicable Date, we had no suspected or confirmed active COVID-19 cases on our premises or among our employees. To prevent any spread of COVID-19 in our offices and production facilities, we have implemented preventive measures such as regularly sterilizing and ventilating our offices and production facilities, checking the body temperature of our employees daily, keeping track of the travel history and health conditions of employees,

SUMMARY

and providing face masks and disinfectant to employees attending our offices and facilities. We will continue to implement our remedial measures and may implement additional measures as necessary to ease the impact of the COVID-19 outbreak on our operations. However, we cannot guarantee you that the COVID-19 pandemic will not further escalate or have a material adverse effect on our results of operations, financial position or prospects.

U.S.-China Relationship

In light of the current situations and the particular nature of the biopharmaceutical industry, we are of the view that the U.S.-China tension has not had any material impact on our business or operations, our clinical trial designs and execution, patient enrollment, data transfer, related regulatory approval processes, ability to find alternative suppliers to source, develop and manufacture our pipeline products, and prospects. We cannot guarantee, however, that the U.S.-China tension will not escalate which may have a material adverse effect on our results of operations.

No Material Adverse Change

Save as otherwise disclosed above, our Directors confirm that, as of the date of this Document, there has been no material adverse change in our financial or trading position, indebtedness, mortgage, contingent liabilities, guarantees or prospects since June 30, 2021, the end of the period reported on in the Accountants’ Report set out in Appendix I to this document. As we continue to advance the development of our pipeline and expand our clinical development programs, we expect to incur increasing research and development expenses and administrative expenses. Therefore, based on the assumptions made by and information currently available to our management, we expect to incur an increased amount of losses in 2021 compared to 2020.

RISK FACTORS

We believe that there are certain risks involved in our operations, many of which are beyond our control. These risks are set out in the section headed “Risk Factors” in this document. Some of the major risks we face include:

- The actual market size of our drug candidates might be smaller than expected and our future approved drug candidates may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.
- Our business and financial prospects depend substantially on the success of our clinical stage and pre-clinical stage drug candidates. If we are unable to successfully complete their clinical development, obtain relevant regulatory approvals or achieve their commercialization, or if we experience significant delays in any of the foregoing, our business and profitability may be adversely affected.

SUMMARY

- We may not be able to identify, discover or in-license new drug candidates, and may allocate our limited resources to pursue a particular candidate or indication and fail to capitalize drug candidates or indications that may later prove to be more profitable, or for which there is a greater likelihood of success. Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies and trials and non-head-to-head analyses may not be predictive of future trial results. As such, we may not be able to successfully expand our drug portfolio, which could materially and adversely affect our future growth and prospects.
- If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.
- We have incurred significant net losses and net operating cash outflows since our inception, and expect to continue to incur net losses and net operating cash outflows for the foreseeable future and may not be able to generate sufficient revenue to achieve or maintain profitability. Potential [REDACTED] are at risk of losing substantially all of their [REDACTED] in our Shares.
- Our rights to develop and commercialize some of our drug candidates are subject to the terms and conditions of licenses granted to us by others.
- Even if we are able to commercialize any approved drug candidates, the drugs may become subject to national or other third-party reimbursement practices or unfavorable pricing regulations, which could materially and adversely affect our business.
- All material aspects of the research, development, manufacturing and commercialization of pharmaceutical products are heavily regulated and the approval process is usually lengthy, costly and inherently unpredictable. Any failure to comply with existing or future regulations and industry standards or any adverse actions by the drug-approval authorities against us could negatively impact our reputation and our business, financial condition, results of operations and prospects.
- We had net current liabilities and net liabilities during the Track Record Period, which may expose us to liquidity risk.
- We have a limited operating history, which may make it difficult to evaluate our current business and predict our future performance.