GLOSSARY OF TECHNICAL TERMS

This glossary contains definitions of certain terms used in this document in connection with us and our business. Some of these may not correspond to standard industry definitions.

"**6MWT**"	The six-minute walk test (6MWT), a functional test that measures the distance an individual is able to walk over a total of six minutes on a hard, flat surface. The goal is for the individual to walk as far as possible in six minutes
"**AAV**"	Adeno-associated virus which has emerged as an attractive vector for gene therapy to promote sustained gene expression in a variety of tissues such as muscle, eye, brain, liver, and lung
"**AAV capsid platform**"	A tool that applies specific virus to investigate targeted treatment
"**aHUS**"	Atypical Hemolytic Uremic Syndrome
"**ALGS**"	Alagille syndrome, is a rare genetic disorder that can affect multiple organ systems of the body including the liver, heart, skeleton, eyes and kidneys
"**AMD**"	Acid maltase deficiency, also called Pompe Disease or Glycogen Storage Disease type II
"**ANCOVA**"	Analysis of covariance
"**APG101**"	Former name of CAN008 before in-licensing
"**ASBT**"	Apical sodium-dependent bile acid transporter
"**ASGCT**"	American Society of Gene & Cell Therapy
"**ATG-012**"	The initial scaled-down batch of CAN106
"**BA**"	Biliary atresia
"**Biomarker study**"	A Study aims to achieve the streamlining of drug discovery and development by investigating the biomarkers
"**BsAb**"	Bispecific antibody

GLOSSARY OF TECHNICAL TERMS

"**C5**"	Complement component 5, a protein that is encoded by the C5 gene
"**CD95**"	FasR/apoptosis antigen 1 (APO-1)
"**CD95L**"	CD95 ligand/FasL
"**CDE**"	Center for Drug Evaluation
"**CDMO**"	Contract Development Manufacture Organization
"**cGMP**"	Current Good Manufacture Practices
"**CHARD**"	China's Alliance for Rare Disease (中國罕見病聯盟), a medical research alliance dedicated to the study of rare diseases in China
"**CHO**"	Chinese hamster ovary cell
"**CMC**"	Chemistry, Manufacturing, and Controls
"**CNS**"	Central nervous system
"**Cohort**"	A group of patients as part of a clinical study who share a common characteristic or experience within a defined period and who are monitored over time
"**CRISPRs**"	Clustered Regularly Interspaced Short Palindromic Repeats
"**CRO**"	Contract research organization
"**DG44**"	A CHO-DG44 subcell line derived from the original CHO-K1 cell line
"**DLT**"	Dose-limiting toxicity
"**DMD**"	Duchenne Muscular Dystrophy
"**DSMB**"	Data and Safety Monitoring Board
"**EC**"	Ethics committees

GLOSSARY OF TECHNICAL TERMS

"**ELISA**"	Enzyme-linked immunosorbent assay, is a commonly used analytical biochemistry assay
"**EMA**"	European Medicines Agency
"**ERT**"	Enzyme replacement therapy
"**EURORDIS**"	European Organization for Rare Diseases
"**FcRn**"	Neonatal Fc receptor, functions as a recycling or transcytosis receptor that is responsible for maintaining IgG and albumin in the circulation
"**FD**"	Fabry disease
"**FDA**" or "**US FDA**"	The United States Food and Drug Administration, a federal agency of the Department of Health and Human Services
"**FGF23**"	Fibroblast growth factor 23
"**GAA**"	Acid α-Glucosidase
"**GAG**"	Glycosaminoglycans
"**GBA**"	The gene encoding beta-glucosylceramidase/beta-glucocerebrosidase
"**GBM**"	Glioblastoma multiforme
"**GC**"	Green Cross Corporation
"**GCase**"	Glucocerebrosidase
"**GD**"	Gaucher Disease
"**GLA**"	Alpha-galactosidase
"**Glycosylation**"	The reaction in which a carbohydrate is attached to a hydroxyl or other functional group of another molecule in order to form a glycoconjugate. It usually refers to an enzyme-catalysed reaction, whereas glycation may refer to a non-enzymatic reaction in biology

GLOSSARY OF TECHNICAL TERMS

"**gMG**"	Generalized myasthenia gravis
"**GOI**"	Gene of interest
"**HER2**"	Human epidermal growth factor receptor 2
"**HSA**"	Health Sciences Authority, the national authority regulating health products in Singapore
"**HSCT**"	Hematopoietic stem cell transplantation
"**humanized**"	Humanized monoclonal antibodies refer to antibodies that are constructed by combining the complementarity-determining regions (CDRs) of the monoclonal antibody with human framework and constant regions, in which the human framework regions were chosen to maximize homology with the monoclonal antibody sequence
"**ICONIC**"	A Phase 2b placebo-controlled randomized drug withdrawal clinical trial on maralixibat conducted for ALGS by Mirum
"**IDS**" or "**rhIDS**"	Iduronate-2-sulfatase
"**INDIGO**"	A Phase 2 study on maralixibat conducted for PFIC by Mirum
"**IND**"	Investigational new drug or investigational new drug application, also known as clinical trial application in China
"**LSD**"	Lysosomal storage disorders
"**Lyso-GL-1**"	Glucosylsphingosine, the deacylated form of glucosylceramide (GL1)
"**M6P**"	Mannose-6-phosphate
"**mAb**"	Monoclonal antibodies
"**MAC**"	Membrane attack complex
"**MAD**"	Multiple ascending dose

"**MG**"	Myasthenia Gravis
"**MGMT**"	O-6-methylguanine-DNA methyltransferase
"**mITT**"	Modified Intention to Treat
"**MMA**"	Methylmalonic acidemia
"**MPS disorder**"	Mucopolysaccharidosis, a group of inherited metabolic disorders in which the body is unable to properly breakdown mucopolysaccharides (long linear polysaccharides consisting of repeating disaccharide units)
"**MPS II**" or "**Hunter Syndrome**"	Mucopolysaccharidosis type II, the most prevalent MPS disorder, occurring in an estimated 1.07/100,000 in newborn
"**NHFPC**"	National Health and Family Planning Commission (國家衛生和計劃生育委員會), a cabinet-level executive department under the State Council which is responsible for providing information, raising health awareness and education, family planning, ensuring the accessibility of health services, monitoring the quality of health services provided to citizens and visitors in China, which has been merged into the National Health Commission (國家衛生健康委員會)
"**NIFDC**"	National Institutes for Food and Drug Control
"**NMO**"	Neuromyelitis Optica
"**NMOSD**"	Neuromyelitis Optica Spectrum Disorders
"**NMPA**"	The National Medical Products Administration (國家藥品監督管理局), which is in charge of comprehensive supervision on the safety management of food, health and cosmetics and is the competent authority of drug regulation in China
"**NOD**"	Nonobese diabetic
"**NRDL**"	National Reimbursement Drug List

GLOSSARY OF TECHNICAL TERMS

"**ODD**"	Orphan drug designation
"**PCT**"	Patent Cooperation Treaty
"**PFIC**"	Progressive familial intrahepatic cholestasis
"**PFS**"	Progression-free survival
"**PHEX**"	Phosphate Regulating Endopeptidase Homolog, X-Linked
"**PhIRDA**"	China Pharmaceutical Innovation and Research Development Association
"**PNH**"	Paroxysmal nocturnal hemoglobinuria
"**PD**"	Pharmacodynamics
"**PK**"	Pharmacokinetics
"**PRDL**"	Provincial Reimbursement Drug List
"**PTRS**"	Probability of technical and regulatory success
"**RBC**"	Red blood cells
"**rhGAA**"	Recombinant human acid α-glucosidase
"**RP2D**"	Recommended phase 2 dose
"**rRt**"	Reirradiation
"**RT**"	Radiation therapy
"**SAE**"	Serious adverse event
"**SCID**"	Severe combined immunodeficiency
"**sL65**"	A novel liver-tropic AAV capsid for use in gene editing and gene therapy, which can be a potential treatment of serious diseases of the liver
"**SMA**"	Spinal Muscular Atrophy

"**SMC**"	Safety monitoring committee
"**SRC**"	Scientific Review Committee
"**SRT**"	Substrate reduction therapy
"**TEAE**"	Treatment emergent adverse events
"**TALENs**"	Transcription Activator-like Effector Nucleases
"**TFDA**"	Taiwan Food and Drug Administration (食品藥物管理署), who is in charge of overseeing food, drug, and medical device safety and quality
"**TMDD**"	Target-mediated drug disposition
"**TMZ**"	Temozolomide
"**TNF**"	Tumor necrosis factor
"**t-test**"	Student's t-test, the t-test is any statistical hypothesis test in which the test statistic follows a Student's t-distribution under the null hypothesis
"**TTF**"	Tumor Treating Fields
"**uGAGs**"	Urine Glycosaminoglycans
"**UGIB**"	Upper gastrointestinal bleeding
"**XLH**"	X-linked hypophosphatemia
"**ZFNs**"	Zinc Finger Nucleases