21.06.2021 – 25.06.2021

LUGAR

Videoconferencia

Jornada de innovación abierta con gran multinacional farmacéutica

Anexo I – Partnering interests

ENABLING TECHNOLOGIES / CONCEPTS

- **Cell and Gene Therapy**
- Clinical stage AAV programs for all DA/FAs
- New capsids for tissue targeting, liver-detargeting and increased payload and reduced immunogenicity
- Novel Gene editing technologies (e.g.prime editing)
- Tunability of gene expression (e.g.switches, ZF-TF, CRISPRa/i)
- Novel technology targeting RNA, RNA tx (e.g.siRNA therapeutics for brain)
- Non-viral gene delivery
- RNA delivery technologies (non-liver; e.g.MSD/NS)
- **Biologics**
- Technologies that enable intracellular targeted biologics
- Technologies that enable orally administered biologics
- Next-gen “killer” bi-or multi-specifics
- **Chemistry**
- AI & ML approaches to advancing med-chem, Novel TPD platform capabilities, LMW targeting approaches for RNA
- Novel generative chemistry approaches, Novel automated chemistry technologies

RESPIRATORY

- **Asthma & COPD**
- Reduce the disease burden with disease modifying therapies
- **Sub-specialty Resp**
- Breakthrough therapies for life-threatening in-hospital Respiratory conditions

- Novel targets with disease modification potential in broad range of high unmet need indications, including IPF, sarcoidosis, PAH, cystic fibrosis, bronchiectasis

- **Food allergy**

- Therapies that could reduce risk of anaphylaxis from accidental exposure, enable patients to re-introduce certain foods to their diet, and ultimately to prevent and/or cure food allergy

- **Digital Innovation**

- Digital solutions to address unmet needs in asthma and COPD

NEUROSCIENCE

- **Neurodegenerative Diseases**

- Amyotrophic lateral sclerosis, Parkinson's Disease, Alzheimer's Disease, Huntington's Disease

- Therapies for neuronal protection, restoration or remyelination, e.g. in multiple sclerosis

- Microglia-focused approaches

- **Pediatric Neurological Diseases**

- Monogenetic neurological and neuromuscular diseases

- Gene therapies

- **Psychiatry**

- Refractory psychiatric disorders

- Treatment resistant depression

- Schizophrenia -negative symptoms

- AD/PD psychosis

- **Others**

- Therapies across broader neuro-muscular diseases and muscular dystrophies

- Migraine (novel mechanisms of action)

CARDIOVASCULAR, RENAL AND METABOLISM

- **Heart Failure**

- HFpEF/HF prevention

- Next generation targets and Novel technologies addressing cardiomyopathies and anti-Arrhythmia therapies

- **Metabolic Diseases**

- Interest in novel agents, transformational assets and platforms including cell and gene therapies

- Disease modifying agents in T1/T2DM

- Targeting unmet need in Obesity (>20% WL)

- **Athero & Thrombosis**

- Expand portfolio beyond inclisiranand TQJ230 (Lp(a) with novel approaches to address CVRR

- **Renal**

- Maximize the value of LNP023 in Renal diseases
- Expand into specialty diseases in CKD: nephropathies, lupus nephritis, SA-AKI, ADPKD, Alport
- Interest in renal indications with limited SOC and high unmet need

HF: heart Failure, HFpEF: HF with preserved Ejection Fraction, CVRR: Cardio-Vascular Risk Reduction, Chronic Kidney

Disease (CKD), Autosomal Dominant Polycystic Kidney Disease (ADPKD), Sepsis-Associated Acute Kidney Injury SA-AKI

OPHTHALMOLOGY

- **Retina**

- Innovative treatments addressing high unmet needs in
    - Diabetic retinopathy
    - Early/ intermediate dryAMD
    - Geographic atrophy
- Disease modifying (non a-VEGF) approaches
    - wetAMD, DME

- **Orphan & Rare diseases**

- Explore cell & gene therapy programs in rare indications (Retinitis Pigmentosa, Stargardt'sdisease, etc.)
- Identify potential to address broader target population
- Explore break-through treatments for uveal melanoma (bridge between retina and orphan disease space)

- **Refractive disorders & dry eye**

- Focus on high unmet needand first-in-class, disease modifying medicines for
    - Dry Eye
    - Refractive DisordersPresbyopia, Myopia

- **Digital Innovation**

- Digital solutions to improve treatment outcome while providing better diagnosis, treatment, and access
- Earlier diagnosis
- Monitoring disease progression
- Reduce therapy burden and optimize treatment frequency

IMMUNOLOGY, HEPATOLOGY& DERMATOLOGY

- **Dermatology**
- First in class oral or topical treatments for Psoriasis
- First in class oral, biologic & topical treatments for Atopic Dermatitis
- Oral or Biologic for Hidradenitis Suppurativa, oral treatments for Vitiligo, Alopecia Areata, Acne
- **Rheumatology & Transplant**
- Immune regulators without immunosuppression
- Tolerance induction
- First in class treatments for Spondyloarthropathies, primary Sjögren's Syndrome and Rheumatoid Arthritis, SLE & IBD if truly differentiated (e.g.in pre-determined responder population; barrier function modulation for IBD)
- Transformational Transplant opportunities
- **Hepatology**
- First in class MoAsfor NASH, Cirrhosis, Auto-immune Hepatitis, Acute Alcohol induced Hepatitis, monogenic liver diseases
- Anti-fibrotic therapies, senescence and regeneration/repair mechanisms
- External collaborations to support combination treatment in NASH
- **Osteoarthritis**
- Disease modifying treatments for Osteoarthritis (targeted therapeutics based on MoAswith pleiotropic effects and combination assets for internal pipeline)
- Regenerative approaches for Tendinopathy (LMW programs preferred)

ONCOLOGY

- **Solid Tumors**
- Breast cancer
- CRC
- RCC
- Lung cancer
- Melanoma
- Pancreatic cancers
- Prostate
- **Hematology**
- Acute myeloid leukemia
- MPN-MF/PV

- Myelodysplastic syndrome
- Diffuse Large B cell lymphoma
- Multiple myeloma
- Acute lymphoblastic leukemia
- Sickle cell disease
- Hemophilia
- Immune thrombocytopenic purpura
- Hematopoietic stem cell transplant

- **New Growth Areas**

- DNA Damage/synthetic lethal targeting molecules/platforms, novel targets in breast cancer and hematologic malignancies
- Advanced molecules complementary and orthogonal to internal targeted therapeutics
- Innate immune system targets
- Myeloid targets

- **CAR-T**

- Next generation CAR constructs; manufacturing approaches
- New signaling domains
- Novel CAR-T targets; targets of potential interest include: CD33, CD138 (for MM), CEA, GP3, EpCAM, mutEGFR, GD-2, MUC-1 (for various solid tumors)
- Allogenic/off the shelf CAR-T and TCR technologies
- Next generation adoptive cellular approaches
- Other cell types (e.g., NK, gamma-delta T, macrophages)

PIONNEERAND LEAD THE RADIO LIGAND THERAPY FIELD

- **Innovativetargets/ vectors**

- Identification / characterization of new targets
- Novel Mechanisms of Action with receptor preferentially expressed on tumor cells or microenvironment, but not in normal tissue

- **NewTherapeutic/ diagnosticIsotopes**

- Close partnership with academia and biotech
- Radioactive atom releasing β (Lu) or α (Ac) particles that induce DNA damage to kill tumor cells
- Pioneer and explore other isotopes' potential in RLT

- **Manufacturing / supply excellence**

- To lead Supply & Distribution of RLT technologies
- Digital and automated supply chain and manufacturing processes

- Partner of choice in scarce raw materials and rare isotopes

- **NewChelators/ Linkers& Ligands**

- Chelators/linkers: Small molecular adaptor enabling attachment of isotope to the ligand

- Ligands: Small molecule, peptide or Ab fragment binding with high affinity on target expressed on tumor cells

GENE THERAPIES

- **Rare CNS**

- Rare, monogenic neurodegenerative diseases

- Genetic epilepsies

- Neuromuscular diseases

- **Next Generation Technologies**

- Novel capsids

- Immune modulation technologies

- Re-dosing technologies

- Novel payloads (e.g. regulatory elements, gene-editing strategies, vectorized antibodies)

- **New Growth Areas**

- Gene therapy approaches for:
    - Prevalent neurologic indications
    - Genetically defined cardiomyopathies
    - Liver diseases
    - Diseases targeting muscle

- **Ultrarare**

- AAV gene therapies for ultrarare (10's to 100's of patients worldwide) conditions with no current treatment options

BIOPHARMA

- **Oncology**

- Expand our reach in Oncology biosimilars

- **Immunology**

- Biosimilars to complement our portfolio of immunology treatments

- **New Growth Areas**

- New growth area examples:
    - Neuroscience biosimilars
    - Rare disease biosimilars

- **Endocrinology**

- Biosimilars to complement our growing portfolio in endocrinology

COVID-19

Inbound BD&L opportunities will be prioritized based on the value we can add based on Company's core competencies

1. Late preclinical and clinical-stage direct-acting antiviral assets, including antibodies, with near-term clinical readout, in scope and will be reviewed
2. ARDS, inflammatory response is in scope
3. Manufacturing of biologics or small molecules for companies will be considered on a case-by-case basis
4. Diagnostics are not in scope
5. Vaccines are not currently in scope (exception: Mass Eye and Ear AAV-based vaccine / AveXis)
6. Consumer products (e.g., hand sanitizers, OTC fever reducers) are not in scope
7. Equipment (e.g. ventilators) is not in scope