

# LEAP-BIO

**LEAP-BIO** is a COSME project which aims to **develop IP licensing intermediary services** for early stage assets in **Pharma** and **BIotech**.



This document summarizes the in-licensing interests collected from big pharma and well-funded biotech companies. The interests are divided into 9 main areas: 1) Immunology, Inflammation & Respiratory Diseases, 2) Rare Diseases, 3) Cardiovascular, Renal & Metabolic Diseases, 4) Infectious Diseases & Vaccines, 5) Ophthalmology, 6) Neurologic & Musculoskeletal Diseases, 7) Oncology & Immunology, 8) Others and 9) Enabling Technologies.

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PARTNERSHIP:



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# 1. Immunology, Inflammation & Respiratory Diseases

## 1.1. Autoimmune diseases

- (Primary) Sjögren's syndrome
- Osteoarthritis
- Lupus
- Lupus Nephritis
- Systemic Lupus Erythematosus (SLE)
- Rheumatoid arthritis
- Psoriatic arthritis
- Spondyloarthropathy
- Eosinophilic inflammation

## 1.2. Dermatology

- Inflammatory skin diseases
- Hidradenitis suppurativa
- Scleroderma
- Severe acne
- Atopic dermatitis
- Psoriasis
- Swelling
- Redness
- Itching

## 1.3. Gastrointestinal diseases

- Nonalcoholic steatohepatitis (NASH)
- Crohn's disease
- Ulcerative colitis
- Liver fibrosis
- Liver cirrhosis
- Eosinophilic esophagitis
- Inflammatory bowel disease (IBD) (including fibrostenotic)
- Celiac disease (including RCD2)
- Motility disorders

## 1.4. Respiratory diseases

- Asthma (including severe)
- Chronic Obstructive Pulmonary Disease
- Non-cystic fibrosis bronchiectasis
- Community acquired pneumonia (CAP)-associated complications
- Interstitial lung diseases
- Pulmonary hypertension (group 1 to 5)
- Acute respiratory distress syndrome
- Adjacent rare cardiopulmonary diseases (including progressive fibrotic interstitial lung disease, bronchiectasis, alpha-1 antitrypsin deficiency, others)
- Idiopathic pulmonary fibrosis
- Progressive fibrosing interstitial lung disease
- Cystic fibrosis
- Chronic Rhinosinusitis
- Hypereosinophilic syndrome
- Chronic rhinosinusitis with nasal polyps

## 1.5. Fibrotic diseases

- Idiopathic pulmonary fibrosis
- Renal fibrosis
- Liver fibrosis
- Systemic sclerosis
- Fibrosis in different organs
- Advanced liver fibrosis (stage F3/F4)
- Primary sclerosing cholangitis
- Non-IPF Interstitial Lung Diseases
- Scleroderma

## 1.6. Other immune-related diseases:

- Chronic lung allograft dysfunction
- Hematopoietic stem cell transplant (HSCT)
- Cardiovascular allograft vasculopathy
- Ischemia reperfusion injury
- Maternal-fetal immunology
- B-cell diseases
- Allergy
- Transplant

## Biological processes and targets of interest:

- \* Novel therapeutic targets (in general)
- \* Novel targets to induce tolerance in autoimmunity, e.g. modulation of Mregs, Bregs, tolerogenic APCs
- \* Innate, Adaptive, Fibrosis, Tolerance and Tissue Regeneration Targets
- \* IL-23 pathway
- \* Autoantibody pathway
- \* Immunogenic cell death-related innate immune activation
- \* Autoantibody production via B cell or plasma cell activation
- \* Options for patients with anti-TNF therapy inadequate response
- \* Barrier functions leading to mucosal healing and resolution of inflammation
- \* T-cell biology (including Treg)
- \* B-cell biology
- \* Cytokine signaling
- \* Myeloid cells, neutrophils, and dendritic cells
- \* Fibrosis mechanisms
- \* Checkpoint receptors
- \* Immunometabolism and tolerance
- \* Mechanisms which promote repair and reversal of fibrosis through inhibition of inflammatory responses, protection of epithelium and normalization of fibroblast activation
- \* Non-invasive biomarkers of disease activity and progression, prediction of efficacy and pharmacodynamic response
- \* Tolerizing approaches, broad MOAs with multi-indication potential
- \* Assets and approaches to modulate GI neuroinflammation
- \* Commensal microbiota enhancing approaches
- \* Novel mechanisms and target molecules to regulate function and phenotype in the innate immune system
- \* Immune evasion approaches including universal donor cell and immune cloaking technologies
- \* Information on pathogenic bacteria as new targets of drug discovery for the treatment of microbiome-related diseases
- \* Drug discovery approaches (e.g. target molecules, mechanisms, drug candidates) that can efficiently induce regulatory T cells with specificity for pathogenic antigens and/or injured organs in living bodies
- \* **SLE and IBD:** Targeting undruggable proteins/RNAs with novel small molecule modalities, hijacking endogenous machinery; bio-synthesis using microbes; microbiome as a novel therapeutic modality (including small molecules, LBPs, strain-engineering etc); microbiome as a data source for analyzing disease mechanisms

## 2. Rare Diseases

### 2.1. Rare immune diseases

- Systemic sclerosis
- Systemic sclerosis - scleroderma
- Autoimmune blistering diseases
- Dermatomyositis

### 2.2. Rare metabolic and pediatric diseases

- Rare lipid disorders
- Lysosomal storage disorders
- Leukodystrophies
- Phenylketonuria
- Inborn errors of metabolism
- Organic acidemias - urea cycle disorders
- Achondroplasia
- Other bone disorders

### 2.3. Rare liver diseases

- Primary biliary cholangitis (PBC)

### 2.4. Rare bone disorders

### 2.5. Rare neurological diseases

- Rare neurodegenerative diseases
- Pompe disease
- Dystrophies
- Congenital type 1A
- Duchenne
- Facioscapulohumeral
- Myotonic type 1
- DNA repeat expansion diseases
- Genetic subtypes of Amyotrophic Lateral Sclerosis (ALS)/Frontotemporal disorders (FTD), rare form of Alzheimer's disease (DIAD), Parkinson's disease (PD), Epilepsy and Rare neurometabolic diseases
- Rare neuromuscular diseases
- Angelman Syndrome

### 2.6. Rare cardio and renal diseases

- IgA and other complement mediated nephropathies
- Fabry disease
- Alport syndrome
- Other glomerulopathies
- Polycystic kidney disease
- Other ciliopathies
- Rare inherited dilated
- Arrhythmogenic Cardiomyopathy
- Hypertrophic Cardiomyopathy
- Rare Heart Rhythm Disorders
- Peri-partum Cardiomyopathy
- (Pre-)Eclampsia

### 2.7. Rare hematologic disorders

- Hemoglobinopathies
- Hemolytic anemias
- Complement biology diseases
- Hemophilia
- Immune mediated blood disorders
- Blood cell and bone marrow disorders
- Rare non-malignant hematologic diseases

### 2.8. Rare endocrine disorders

- Rare adrenal
- Pituitary and growth related diseases
- Haemophilia

### 2.9. Rare genetics disorders

- Rare monogenetic diseases

### Biological processes and targets of interest:

- \* Novel therapeutic targets (in general)
- \* Targets related with genetic/rare kidney diseases such as ADPKD, Alport, FSGS
- \* Targeting undruggable proteins/RNAs with novel small molecule modalities, hijacking endogenous machinery; bio-synthesis using microbes; microbiome as a novel therapeutic modality (including small molecules, LBPs, strain-engineering etc); microbiome as a data source for analyzing disease mechanisms
- \* Other innovative therapeutic approaches

## 3. Cardiovascular, renal & metabolic diseases

### 3.1. Cardiovascular diseases

- Myocarditis
- Severe forms of atherosclerosis
- Dilated cardiomyopathy
- Thrombosis
- Thromboembolic diseases
- Acute myocardial infarction
- Portal hypertension
- Hypertension
- Coronary heart diseases
- Hyperlipidaemia
- Atherosclerosis
- Heart failure with preserved ejection fraction (HFpEF)
- Vascular dysfunction
- Lymphatic dysfunction
- Vasculitis
- Stroke
- Myopathies
- Heart failure
- Atherosclerosis
- Acute heart failure
- Chronic heart failure
- Heart failure with preserved ejection fraction
- Atrial Fibrillation
- Multiple cardiovascular risk factor management

### 3.2. Hematology

- Hemorrhagic and acute ischemic stroke
- Acute thrombosis (venous and arterial)
- Sickle cell disease
- Thalassemias
- Benign hematology

### 3.3. Metabolic diseases

- Type 1 diabetes
- Type 2 diabetes
- Obesity
- Mitochondrial dysfunctions

### 3.4. Renal diseases

- Cardio-renal risk-reduction
- Acute kidney diseases
- Chronic kidney disease (incl. anaemia)
- Anaemia of chronic kidney disease
- Polycystic Kidney Disease
- Alport Syndrome
- Focal Segmental Glomerulosclerosis
- IgA Nephropathy
- Diabetic nephropathy

### 3.5. Endocrinology

- Thyroid disorders
- Pediatric growth hormone deficiency
- Growth disorders

### Biological processes and targets of interest:

- \* Novel therapeutic targets (in general)
- \* Drug targets not readily amenable to current approaches
- \* Targets that slow renal decline
- \* Novel targets and /or agents addressing specific cardiomyopathies (e.g., genetically defined and amyloidosis-related)
- \* Targets related with podocyte injuries, impairment of tubular epithelial cells
- \* Glucose-sensing insulins or other insulins with reduced hypoglycemia
- \* Next Generation Immunomodulation
- \* Islet cell and immunotherapies for type 1 diabetes, type 2 diabetes regression and durability of response (e.g. modifiers of beta-cell health)
- \* Protection against or regression of adverse remodeling of the heart, including: fibrosis, hypertrophy, resolution of inflammation, cardiomyocyte preservation or regeneration
- \* Improvement of peripheral vascular compliance
- \* Preservation or improvement of renal function/renal perfusion in heart failure patients
- \* Enhancement of cardiac function, including improvements in contraction or relaxation
- \* Protein homeostasis/degradation, epigenetics, other potentially transformative new targets and pathways
- \* Novel mechanism and target for regulating autophagy
- \* Modulation of mitochondria or related pathways
- \* **Diabetes** - glycemic control
- \* **Heart failure and chronic kidney disease:** Targeting undruggable proteins/RNAs with novel small molecule modalities, hijacking endogeneous machinery; bio-synthesis using microbes; microbiome as a novel therapeutic modality (including small molecules, LBPs, strain-engineering etc); microbiome as a data source for analyzing disease mechanisms

## 4. Infectious diseases & vaccines

### 4.1. Viruses

- Influenza
- Influenza (vaccines)
- Viral hepatitis & adjacent liver diseases
- Respiratory infections (incl. Syncytial)
- Respiratory Syncytial Virus (vaccines)
- HIV
- COVID-19
- COVID-19 (vaccines)
- Hepatitis B
- Adenoviral infections
- Emerging virus threats (vaccines)

### 4.2. Bacteria

- Prevention and treatment of bacterial respiratory infections
- Multidrug-resistant
- Gram-negative bacterial infections
- Gram-negative blood stream infections
- Non-tuberculosis mycoplasma
- Urinary tract infections
- Urogenital gonorrhoea
- Shigella
- Staphylococcus aureus
- Clostridium difficile
- Klebsiella
- Shingles
- Meningitis
- Invasive nontyphoidal salmonella
- Typhoid and paratyphoid fever
- Group A streptococcus
- Diabetic foot infections
- Bone and joint infections
- Combined therapy for gonorrhea and chlamydia
- Bacterial pathogens (e.g. Chlamydia trachomatis, Staphylococcus aureus) (vaccines)

### 4.3. Fungal infections

- Invasive candidiasis

### 4.4. Parasites

- Malaria
- Visceral leishmaniasis
- Chagas disease
- Lymphatic filariasis
- Soil-transmitted parasites
- Dengue fever

\*Vaccines targeting additional conditions could also be considered, especially those that address unmet medical needs.

### Biological processes and targets of interest:

- \* Novel therapeutic targets (in general)
- \* Novel targets in the immune therapy for malaria, HIV and COVID-19
- \* Protective cell mediated response to bacterial or viral infections
- \* Other innovative therapeutic approaches

## 5. Ophthalmology

### Therapeutic indications:

- Retinal disease
- Geographic atrophy
- Wet age-related macular degeneration
- Diabetic macular ischemia
- Diabetic macular edema (DME)
- Diabetic retinopathy (DR)
- Non-proliferative diabetic retinopathy
- Stargardt disease (core)
- Glaucoma
- Retinal degeneration in glaucoma
- Myopic macular degeneration
- Proliferative diabetic retinopathy
- Chronic panuveitis
- Retinitis pigmentosa
- Retinal vein occlusion
- Age-related macular degeneration (AMD)
- Retinal vein occlusion (RVO)
- Dry eye disease

### Biological processes and targets of interest:

- \* Novel therapeutic targets (in general)
- \* Vision restoration
- \* Regeneration of target organ
- \* Other innovative therapeutic approaches

## 6. Neurologic & Musculoskeletal Diseases

### 6.1. Neurodegenerative diseases

- Alzheimer's disease (AD)
- Parkinson's disease (PD)
- Multiple sclerosis (MS)
- Huntington's disease (HD)
- Friedreich's ataxia
- Amyotrophic lateral sclerosis (ALS)
- Myotonic dystrophy
- Frontotemporal dementia (FD)
- Repeat expansion diseases
- Amyotrophic lateral sclerosis
- Involuntary brain diseases

### 6.2. Psychiatry & Mental Health

- Bipolar disorder
- Schizophrenia
- Depression & treatment resistant depression
- Impulse control disorders
- Autism spectrum disorders

### 6.3. Neuroinflammation & Neuroimmune

- Neuromyelitis Optica

### 6.4. Neurological diseases

- Epilepsy
- Drug refractory epilepsy
- Epileptogenesis
- Autoimmune epilepsy
- Peripheral neuropathies
- Hypersomnia

### 6.5. Neuromuscular & Musculoskeletal Diseases

- Movement disorders
- Myasthenia Gravis
- Muscular dystrophies
- Spinal muscular atrophy
- Duchenne Muscular Dystrophy
- Facioscapulohumeral muscular dystrophy
- Myotonic dystrophy type 1
- Sarcopenia (mainly caused by hip fracture or cancer cachexia)

### 6.6. Pediatric Neurological Diseases

### 6.7. Neurogenetics

- Genetic neuropathies

### 6.8. Pain

- Neuropathic pain (diabetics, neuro)
- Chronic neuropathic pain
- Musculoskeletal pain
- Visceral pain
- Migraine

### Biological processes and targets of interest:

- \* Novel therapeutic targets (in general)
- \* Therapeutic targets in the pain field
- \* Targets that modulate protein homeostasis, protein clearance, immune system biology, inflammation and reduce or eliminate toxic protein production
- \* Targets in sporadic and orphan/rare neurological and neuromuscular diseases
- \* Senescence
- \* Aging
- \* Mitophagy
- \* Neuroimmune pathways
- \* Neuron-Astrocyte metabolic coupling
- \* Enhancement of myelination
- \* Suppression of microglia mediated synapse loss
- \* Prevent damage or repair joints in musculoskeletal diseases
- \* Prevention or repair of peripheral nerve damage and neuropathies
- \* Neuroinflammation and therapeutic target engagement

#### Alzheimer's disease:

- \* Neuron-Astrocyte metabolic coupling
- \* Enhancement of myelination
- \* Suppression of microglia mediated synapse loss

#### Multiple sclerosis:

- \* T cell, B cell and Microglia - suppression of immune reactions related to meningeal tertiary lymphoid structures
- \* Oligodendrocyte precursor cell - differentiation to oligodendrocyte and myelination

#### Schizophrenia:

- \* Microglia - suppression of cytokine production and/or phagocytosis
- \* Oligodendrocyte precursor cell - differentiation to oligodendrocyte and myelination
- \* Glutamatergic neuron - amelioration of NMDA signal

#### AD, MS, Schizophrenia and Sarcopenia:

- \* Targeting undruggable proteins/RNAs with novel small molecule modalities, hijacking endogenous machinery; bio-synthesis using microbes; microbiome as a novel therapeutic modality (including small molecules, LBPs, strain-engineering etc); microbiome as a data source for analyzing disease mechanisms

#### Neurodegeneration (AD, PD, ALS, FD and HD):

- \* Protein misfolding (inhibiting intra-neuronal accumulation, aggregation and spreading of misfolded pathological protein species)
- \* Innate immune response and neuroinflammation (enhancing the glial neuroprotective response in neurodegenerative disease)
- \* Reducing inflammation in the brain by modulation of involved pathways, for example microglia, astrocytes, complement cascade
- \* Proteostasis (maintaining protein homeostasis through protein degradation, protein stabilization, protein-protein interaction modulation, autophagy modulation)
- \* Mechanisms of neurodegeneration (blocking pathways that underlie neuro-, axonal- and synaptic degeneration)
- \* Symptomatic treatments (psychosis, cognitive impairment, agitation, mood, movement and sleep)

# 7. Oncology & Immuno-oncology

## 7.1. Solid tumors

- Prostate cancer
- Lung cancer
- Bladder cancer
- Colorectal cancer
- Breast cancer
- Colon cancer
- Non-Small Cell Lung Cancer (NSCLC)
- Non-melanoma Skin Cancers
- Gynaecologic cancers
- Lung cancer
- Ovarian cancer
- Liver cancer
- Pancreatic cancer
- Prostate cancer
- Other solid tumors

## 7.2. Hematologic malignancies

- Multiple Myeloma
- Lymphoma
- Chronic lymphocytic leukemia
- Myelodysplastic syndromes
- Acute myeloid leukemia
- Myeloproliferative neoplasms
- Myelofibrosis
- Other hematologic malignancies

## 7.3. Immuno-oncology

### Biological processes and targets of interest:

#### Novel therapeutic targets:

- \* In immune oncology, incl. innate immune cell targets
- \* In colorectal, breast, colon, lung, prostate, pancreatic cancers
- \* Related to genomic instability/mutation
- \* Oncogenic transcription factors and their complexes
- \* Tumor myeloid and stromal factors
- \* Resistance pathways
- \* Tumor drivers and resistance
- \* Others

#### T-cell engagers and immune cell engagers:

- \* New targets for T-cell engagers, PD-1 betterers, and novel checkpoints
- \* Next-gen innate cell engagers
- \* Bi/trispecific T/NK cell engagers
- \* Bispecific immune cell engager

#### Tumor microenvironment and stromal biology:

- \* Profiling of tumor myeloid cells to find novel targets
- \* Inhibiting myeloid suppressor cells, and enhancing macrophage and dendritic cell activity
- \* Tumor stromal biology
- \* Modulation of macrophages and/or neutrophils

#### ADCs (antibody-drug conjugates) and payloads:

- \* Novel ADC targets
- \* Improved tumor-selective targeting
- \* Novel payloads and designs

#### DNA damage, repair, and instability:

- \* Oncogenic signaling, DNA Damage & Repair, Tumor Stress & Plasticity
- \* Synthetic lethality
- \* Focal cytotoxics
- \* DNA damage response
- \* Chromosomal instability

#### Immune modulation and response:

- \* Novel activation of nucleic sensing
- \* Resistance to Immune Checkpoint Inhibitors
- \* Antigen presentation, MHC modulation
- \* Nonsense Mediated Decay
- \* Immunoproteasome
- \* Tumor intrinsic biology with clear patient selection strategy
- \* Novel innate and adaptive immune mechanisms
- \* Novel mechanisms addressing primary or acquired resistance to cancer immunotherapy
- \* Novel mechanisms complementary to anti-PD-1 or addressing unmet needs in various malignancies
- \* Adaptive and innate immunity
- \* Neoantigens

#### Targeted drug delivery:

- \* Surface antigens for tissue-specific drug delivery and screening techniques

#### Other innovative technologies:

- \* Multi-specific antibodies enabling unique immune control



## 8. Others

### 8.1. Women's health

- Fertility
- Contraceptives
- Pregnancy management
- Vitamins
- Nutritional needs
- Menopause
- Vaginal infections
- Care of intimate area

### 8.2. Aesthetics

- Hair growth

### 8.3. Ear diseases

### 8.4. Geriatric medicine

- Age-related disorders

### Biological processes and targets of interest:

- \* Novel drug target/mechanisms (not published) for age-related disorders
- \* Regeneration of hair cell and repair of ribbon synapse formation
- \* Strial vascular function improvement (preferably lead molecule identified)
- \* Senomorphic/Senolysis/Rejuvenation/SASP
- \* Senescence

# 9. Enabling Technologies

## 1. Drug Discovery and Development

- Innovative animal models, particularly for infectious diseases
- Animal models for pharmacologic pain
- Preclinical in vitro models that are more translated to the human body
- iPSC-based models
- Innovative human tissue/organ models for translational screening or characterization of AAVs
- Technologies for phenotypic screens (including target deconvolution)
- Novel screening platforms
- In silico platforms for small molecule drug discovery
- Technologies that accelerate drug discovery and development
- Platforms that generate drugs with similar mechanism of actions of immunoglobins and antibodies
- Chemistry platforms, bioprocesses, measure toxicology and safety profiles
- Novel technologies supporting clinical development of new heart failure therapies
- Emerging protein structure determination platforms
- Microfluidics based platforms
- Translationally relevant preclinical models
- Systems biology tools to evaluate pharmacologic/toxicologic responses
- Label-free cellular target engagement platforms
- Novel HTS/lead generation approaches
- Novel chemical libraries with evidence of biological relevance New systems for drug targeting
- Predictive safety and predictive efficacy platforms
- Novel CMC, manufacturing and analytical processes
- Screening technologies or novel assays for autophagy regulator
- Screening platform to identify direct reprogramming factors
- Technology for recognizing environment-specific target antigens and proteins in oncology
- Ophthalmology: technology to improve CMC of cell, gene and extracellular vesicle (EV), Safer and efficient AAV serotypes for subretinal and intravitreal injection
- Ear diseases: Animal models to predict human effectiveness in sensorineural hearing loss; Animal model for microbiome-related diseases

## 2. Drug Delivery Systems and Routes

- New delivery systems
- Non-viral in vivo delivery of RNPs
- mRNA and lipid nanoparticle platforms
- Formulations and delivery routes for mRNAs (lipid nanoparticles or polymers)
- New technologies for intracellular delivery of antibodies
- Targeted-delivery technologies to specific cells/tissues, including gut, liver, lung, kidney, immune cell subsets, central nervous system, muscle
- Deliver technologies for RNA, peptides, proteins and gene therapy
- Nanocarriers, conjugated delivery, AAVs, and delivery platforms that can enable new routes of administration
- Controlled release technologies for drug delivery
- Injector devices
- Formulation and drug delivery technologies for oral delivery
- Invasive device technologies for cell therapy

- Cell-penetrating technologies, including exosomes and others
- Technologies that translocate the BBB
- Technologies directed toward enhancing GI absorption of poorly absorbed compounds or enabling novel delivery methods (colonic, intraoral, subcutaneous, intra-tumoral)
- AAV constructs and viral vectors
- Biodistribution, tissue tropism, drug-to-target enhancement, CNS/PNS

### Rare Diseases:

- AAV with improved tropism for specific organs, including neuromuscular disorders
- Promoters with context dependent efficiency
- Alternative delivery systems that enable re-administration or treatment of pediatric patients

### Neuroscience:

- AAV capsids for intrathecal or systemic administration, with widespread or region/cell specific transduction and minimal DRG impact

### Renal Disorders:

- Nanoparticle delivery to the kidney and kidney specific compartments and cell types, AAV Tropism specific to kidney

### Rare Genetics & Hematology:

- Non-viral gene therapy delivery technologies that allow re-dosing

### Neuroscience:

- Enhanced BBB and skeletal muscle RNA delivery technology
- Non-viral and viral gene delivery to brain, muscle

### Ophthalmology:

- Technology to enable less-invasive and efficient delivery of drugs into the eye

### Ear diseases:

- Drug delivery system that targets the inner ear with small compound/protein/nucleic acid/gene therapy
- Linear mRNA/plasmid that can load multiple genes in tandem, circular RNAs that can load multiple gene, or others
- Lipid nanoparticles or others capable of efficiently delivering mRNA/plasmid to fibroblasts

## 3. Biotherapeutic Technologies

- Protein purification technologies, bioprocess improvements, and MDCK cell culture yield improvements
- Innovative research addressing improved delivery, formulation, stabilization (5°C / room temperature), shelf-life extension and manufacturing technologies
- Technologies for half-life extension systemically and in the eye
- Cell encapsulation technologies
- Peptide and protein technology platforms (expression, purification and modification)
- Antibody drug conjugates, conjugation and encapsulation approaches allowing targeting to specific tissue
- Solid state stabilization of proteins to enable high-concentration parenteral delivery
- Technologies that can enhance internalization and trafficking to lysosomes

#### 4. Adjuvants, Antigens, and Vaccine Technologies

- Influenza virus antigen purity and yield enhancement
- Proven adjuvant technology
- Research related to the adjuvant MF59®
- Viral vectors & adjuvants & vaccine technologies
- Novel delivery, manufacturing and analytical processes
- Technologies reducing pre-existing immunity for AAV gene therapy approaches

##### Infectious Diseases:

- mRNA vaccine technologies (mRNA, delivery, stabilization, production and formulation)
- mRNA vaccine raw materials and production (pDNA, improved enzymes, lipids)
- Novel antigens and methods for antigen discovery, optimization and characterization
- New ways to administer vaccines, including mucosal routes (oral, sublingual, intranasal)
- Nanoparticles, carrier proteins, and methods of conjugations of proteins and polysaccharides
- Novel vectors for delivering antigens
- Adjuvants and immunomodulators
- Vaccine manufacturing (Prokaryotic or eukaryotic cell lines for antigen production, Upstream and downstream processes optimization technologies, Process automation and digital innovation, Preservatives and stabilizers, Nonionic detergents, Anti-counterfeiting technology)
- Microbiome Associated Technologies (Biologics (antibodies, phages, etc.) to modify the GI, skin, and/or oral microbiome)

#### 5. Biomarkers and Patient Stratification

##### Oncology and Immuno-oncology:

- Biomarker and technology platforms for the advancement of cancer immunotherapies
- Translational datasets (longitudinal, transcriptomic samples from SOC experienced patients)

##### Immunology:

- Precision medicine/patient stratification approaches
- Identification, characterization and validation of biomarkers for patient stratification and monitoring of clinical responses using a precision medicine approach
- Biomarkers of disease activity to inform patient stratification, measure pharmacodynamic responses and predict efficacy, with a particular interest in such biomarker-enabled programs

##### Neuroscience:

- Biomarkers predictive of disease progression, treatment response and patient stratification, PET ligands for misfolded proteins
- Translational tools and technologies such as neuroimaging and fluid biomarkers to track neurodegenerative disease
- Super resolution imaging platforms
- Longitudinal patient data sets, biomarkers, imaging tools

##### Gastroenterology:

- Targeted-delivery technologies to gut or liver, including cell-specific approaches
- Single-cell profiling technologies
- Translational patient datasets
- Other patient stratification approaches and biomarkers

#### 6. Genomic Medicine and Gene Editing

- Gene correction/replacement
- Epigenetic editing
- RNA engineering technologies (e.g. UTRs, IRES, circular RNA, chemical modifications, stability)
- Regulatable gene expression
- Next-generation gene editing
- Genetic medicine platforms
- Single cell genomic and proteomic platforms
- CRISPR technologies
- Novel technologies for gene regulation and RNA editing
- Technologies to enable AAV re-dosing and treatment of patient with pre-existing immunity
- Improved AAV manufacturing processes
- Gene therapy enhancements: transgene & promoter engineering

##### Immunology:

- Analysis technologies for immune microenvironment including single cell analysis and spatial gene expression
- Genetic engineering approaches for controllable gene expression to enhance maturation and/or therapeutic effects of our cells
- Genome editing and modification technology in bacteriophage

#### 7. Artificial Intelligence, Machine Learning and Digital technologies

- in-silico and AI discovery and optimization platforms for biotherapeutics, biologic enabling technologies
- Techs for increasing speed / efficiency of medchem work: AI for multi-parameter optimization
- Technologies for lab automation
- 3D bioprinter, intelligent image analysis tools, tissue imaging and real-time single cell sorting/purification based on machine learning
- Machine learning capabilities applied to research and early development
- Companion digital therapeutics that enhance delivery of care
- Artificial intelligence for target and drug discovery/development