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 & Immuno-oncology, 8) Others and 9) Enabling Technologies.

If you have any questions, please contact imm-techtransfer@medicina.ulisboa.pt

PARTNERSHIP:

Funded by the European Union. Views and opinions expressed are however those of the author(s) only and do not necessarily refl ect those of the European Union or European Innovation Council and SMEs Executive Agency (EISMEA). Neither the European Union nor the granting authority can be held responsible for them.

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 dermatathis
- 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 hyoertension (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 scleroderma
- 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 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

2. Rare Diseases

2.1. Rare immune diseases

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

2.2. Rare metabolic and pediratric 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 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
* 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 retinophaty (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
- Involutional 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

- Neuropatic pain (diabetics, neuro)
- Chronic neuropathic pain
- Musculoskeletal pain
- Visceral pain
- Migrane

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 musculosketetal 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 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

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 betters, 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 intravitreous injection
- Ear diseases: Animal models to predict human effectiveness in sensorineural hearing loss; Animal model for microbiome-related diseases

2. Drug Dilevery 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 Tecnologies

- 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