

A1 Oncology

Immunooncology

A1-1	<p>Novel targets or research on tumor immunotherapy to overcome ICI insensitivity or resistance</p> <p>Focused cell types or mechanisms:</p> <ul style="list-style-type: none">•Suppressive immune cells<ul style="list-style-type: none">MDSCsInnate lymphoid cells (ILCs)•Tumor cells<ul style="list-style-type: none">Enhancement of tumor antigenicity•T cell dysfunction including T cell exhaustion
------	--

Disease model for pancreatic cancer

A1-2	<p>Pancreatic ductal adenocarcinoma (PDAC) models which reflects human PDAC features, and better than known genetically engineered mouse models (GEMM) like KPC (KRAS, TP53) in some points of following features</p> <ul style="list-style-type: none">•High desmoplasia and stroma content•low vasculature that reflects human PDAC•established methodology to monitor PDAC tumor growth in pancreas•possible strategy and plans to monitor antibody distribution to PDAC in pancreas
------	--

Antibody-related research

A1-3	<p>Unique antibodies or binders, applicable to mono- or multi-specific antibodies and/or cell therapy, against tumor-associated antigens, which include tumor, immune, and stromal cell targets.</p> <ul style="list-style-type: none">•Binders include antibody mimetics, Fab, scFv, sdAb.
------	---

Cell therapy

A1-4	<p>Novel technologies for adoptive T cell therapy</p> <ul style="list-style-type: none">•Novel molecular targets/mechanisms to potentiate T-cell functions•Novel molecular targets/mechanisms to enhance efficacy in solid tumor•Novel conditional activation machinery at tumor sites (On/Off switch etc.)
------	---

B1 Immune related disease

Immune diseases/fibrosis

B1-1	<p>Novel therapeutic targets or novel target identification involved in refractory immune diseases or fibrosis. New targets are those that have not been tested in clinical trials and may also be known molecules.</p> <ol style="list-style-type: none">1) Novel cell surface targets2) Novel intracellular targets <p>If the target has been acquired,</p> <ul style="list-style-type: none">•The targets can be either specific subset of pathogenic cells or molecules involved in these disease mechanisms.•The targets are desired to be scientifically validated in a preclinical study.•For cell surface targets, antibodies have been obtained <p>In the case of target identification studies,</p> <ul style="list-style-type: none">• An exploratory study of novel therapeutic targets utilizing patient sample collection and omics data analysis before and after administration of existing therapeutic agents.•Transcriptome analysis (scRNA-seq, CITE-seq, LIBRA-seq, and Spatial transcriptomics), GWAS and/or PheWAS analysis or whole exome analysis from human samples.
------	---

Evaluation technology

B1-2	<p>Novel drug target validation tools which mimicks pathological mechanism of refractory immune-inflammatory disorders (such as pulmonary fibrosis diseases, interstitial lung disease, and autoimmune disease with neuropsychiatric symptoms)</p> <ul style="list-style-type: none">•Novel validation tools using patient cells-derived iPS cells and organoids•Novel validation tools using Organ on a chip technology•Novel animal models•Validation of drug targets using above tools
------	--

B2 CNS

Psychiatric disease

Unique research on psychiatric diseases which can pave the way to novel drug development.

• Focused diseases: Major Depression Disorder, Anxiety disorder, Schizophrenia, Autism Spectrum Disorder

(B2-1) Neuroinflammation

Novel target and psychiatric disease animal model induced by primary neuroinflammation, with human translatability by biomarker, suitable to judge the therapeutic potential of drug or target.

B2-1

B2-2

B2-3

(B2-2) New targets

Research for finding brand new therapeutic target to tackle UMN of psychiatric disease based on human disease information.

(B2-3)

Research for endogenous ligand, receptor, or pathway which is expected to be involved in abnormal activity of specific brain area causing psychiatric disease. Abnormal activity of specific brain area reported in patient and reproducibility in rodent model is recommended.

Neurodegenerative disease

Unique research on neurodegenerative diseases which can pave the way to novel drug development.

• Focused diseases: Alzheimer's Disease, Progressive Supranuclear Palsy, Frontotemporal Lobar Degeneration, Parkinson's Disease, Multiple System Atrophy, Dementia with Lewybody, Amyotrophic Lateral Sclerosis

(B2-4) Neuroinflammation

Novel research on glial cell function which can reveal relevance of the progression of neurodegenerative diseases. Ideas with high originality regarding neuroinflammation, cellular metabolism and senescence are of particular interest.

B2-4

B2-5

B2-6

B2-7

(B2-5) New targets

Research for finding novel therapeutic target utilizing clinical information regarding prognosis and/or data from clinical trials.

As an example, research to identify new drug targets based on the clinical information that thin people progress faster in ALS and that calorie intake is the standard of care in the early stages of the disease.

(B2-6) Novel research which focuses on neuroprotection to prevent disease progression. In addition to approach for direct neuroprotective effects, approach to elucidate novel, glial cell-mediated neuroprotective mechanism will be highly valued.

(B2-7) Disease model

Translational disease model of neurodegenerative diseases.

B3 Organ protection

NASH

B3-1	Novel therapeutic target for NASH and/or target identification for NASH based on unique screening platform Out of scope: MOA reducing TG in liver • Exploratory research to identify novel therapeutic target for NASH based on unique screening platform using iPS cells or organoid derived from NASH patients
------	--

Chronic Heart Failure

B3-2	Innovative research to provide novel therapeutic targets for HFREF, HFpEF using human myocardial samples or organoid derived from HF patients
------	---

B4 Ophthalmic Disease

Age-Related Macular Degeneration

B4-1	Research on Drug Target Molecules/Mechanism for intermediate or non-exudative age-Related Macular Degeneration. • Limitation: The target molecules will be limited to those with proven efficacy in animal models or those expected from analysis of patient samples.
------	--

Retinitis Pigmentosa

B4-2	Research on Drug Target Molecules/Mechanism for Gene-independent Therapy of Retinitis Pigmentosa. • Limitation: The target molecules will be limited to those with proven efficacy in multiple animal models or those expected from analysis of patient samples.
------	---

B5 Mechanism based strategy

Gene therapy

B5-1	Novel target genes whose expression needs to be repressed for therapeutic purposes (Our focus is CNS, but the scope is not limited to CNS. Our scope also includes liver, muscle, heart, pancreas and so on) Novel secreted factors, such as proteins, peptides, or biologics which can be delivered by gene therapy vector for therapeutic purposes
------	---

mRNA-related research

B5-2	Non-coding RNA or NMD-sensitive mRNA which work as therapeutic target Especially, we are interested in those targeted with oligonucleotides by following mechanisms: • Convert endogenous non-coding RNA or NMD-sensitive mRNA into functional RNA by either changing a splice site or RNA editing • Block function of non-coding RNA by either changing a splice site or RNA editing Out of scope: • Micro RNA • Therapeutic targets for cancer
------	--