Needs & wants List（Intractable / Rare diseases）

Diseases	• Intractable and rare diseases ➢ Intractable disease: no existing treatment or no response ➢ Rare disease: prevalence rate <0.1%
Object	• Drug Discovery Seeds • Drug targets and concepts • Technology that can be utilized for drug discovery
Seeds	Small molecules or peptides that satisfy either of the following requirements: • Demonstrate a direct effect on the cause of the disease in vitro or be strongly expected to do so. • Demonstrate an ameliorating effect in an animal model.
Targets	1) Unpublished targets that satisfy either of the following requirements: ➢ Novel causative genes or targets that are strongly expected to be involved in the progression of the disease condition. (Desirable to be demonstrated POC in vivo) ➢ Concepts that are strongly expected to lead to improvement in QOL or physical function 2) Targets for the treatment of immune disorders regardless of prior publication that satisfy either of the following requirements: ➢ Targets for the treatment of autoimmune diseases and autoinflammatory diseases. (Desirable to be able to differentiate from existing therapies) ➢ Targets for unmet medical needs that improve the disease condition or symptoms
Related technologies	• Technologies to evaluate new or less utilized indexes (such as phenotype) ➢ Animal disease models that reflect the human clinical condition ➢ Cell-based assays that reflect the disease condition • Technologies to deliver drugs to specific organs • Technologies to increase the transfer of drugs to the brain

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