

Needs & Wants List (Intractable and Rare Diseases)

Diseases	<ul style="list-style-type: none">• Intractable and rare diseases Intractable disease: no existing treatment or no response Rare disease: prevalence rate <0.1%
Object	<ul style="list-style-type: none">• Drug discovery seeds• Drug targets and concepts• Technology that can be used in drug discovery
Seeds	Small molecules, peptides, and other modalities that satisfy either of the following requirements: <ul style="list-style-type: none">• 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Targets	The research must meet one of the following criteria (preferably unpublished) <ul style="list-style-type: none">• A novel causative gene or a target that is expected to be strongly involved in the progression of the disease (in vivo POC should be confirmed).• Drug concept that is expected to improve the prognosis of the disease or the patients' daily activities.• Discovery concepts that can be differentiated from existing therapies.
Related technologies	<ul style="list-style-type: none">• 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 <ul style="list-style-type: none">• Technologies to deliver drugs to specific organs• Technologies to increase the transfer of drugs to the brain• Technologies for supplementing genes using non-viral methods

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