

Needs & wants List (Intractable / Rare diseases)

Diseases	<ul style="list-style-type: none"> • Intractable and rare diseases (except for cancer, infections) <ul style="list-style-type: none"> ➤ Intractable disease: no treatment exists ➤ Rare disease: prevalence rate <0.1%
Object	<ul style="list-style-type: none"> • Seeds • Targets, concepts • Technologies
Seeds	<p>Small molecules or peptides that satisfy either of the following requirements:</p> <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	<p>1) Unpublished targets that satisfy either of the following requirements:</p> <ul style="list-style-type: none"> ➤ New causative genes or targets that are strongly expected to be involved in the progression of the disease condition. (Desirable to demonstrate POC in vivo) ➤ Concepts that are strongly expected to lead to improvement in QOL or physical function <p>2) Targets for the treatment of immune disorders regardless of prior publication that satisfy either of the following requirements:</p> <ul style="list-style-type: none"> ➤ Targets for the treatment of autoimmune diseases and autoinflammatory diseases ➤ Targets for unmet medical needs that improve the disease condition or symptoms
Related technologies	<p>Technologies to evaluate new or less utilized indexes (such as phenotype)</p> <ul style="list-style-type: none"> • 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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