

Needs & Wants List (Nucleic acid drugs)

Diseases	Diseases amenable to treatment by nucleic acid drugs
Object	<ul style="list-style-type: none">• Targets• Concepts• Technologies that can be used in drug discovery
Targets	Targets amenable to nucleic acid drugs
Concepts	RNAi, exon skipping, aptamers, cleavage by nuclease (Gapmer) and/or new concepts that follow on from the foregoing (preferably unpublished)
Related technologies	<ul style="list-style-type: none">• Nucleic acid DDS technology Technology to improve kinetic properties such as blood retention, tissue specificity and directionality (especially in skeletal muscle and central nervous system), and intracellular transfer Technology to enable oral administration of nucleic acid drugs• Modified nucleic acid technology Technology related to new modification of oligonucleic acid and new skeleton (that can improve drug efficacy, reduce toxicity, and improve kinetics)• Novel Target Discovery Technology A genetic analysis technology capable of identifying novel target molecules suitable for nucleic acids. Methods capable of analyzing human and patient samples are desirable.

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Needs & Wants List (Gene therapy)

Diseases	Diseases amenable to treatment by in vivo gene therapy (excluding cancer viral therapy)
Object	<ul style="list-style-type: none">• Drug Discovery Seeds• Targets• Concepts• Technologies that can be used in drug discovery
Seeds	Gene therapy products for central nervous system diseases, ophthalmic diseases, or muscle diseases
Targets	Novel targets suitable for gene therapy
Concepts	Gene replenishment for defective genes and/or new concepts that follow on from the foregoing
Related technologies	<ul style="list-style-type: none">• Technologies related to AAV Technologies contributing to the expansion of AAV vector applications, such as giant gene delivery technology• Technologies other than AAV Viral or non-viral vectors that can be applied to gene therapy• Technologies that can provide innovative value in gene delivery and expression control, not limited to the above (having advantages over other technologies are desirable)

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