

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
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)

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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 or muscle diseases
Targets	Novel targets suitable for gene therapy (especially in the area of central nervous system diseases)
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 <ul style="list-style-type: none"> ➢ Tissue/cell-directed modified capsid, technology to carry giant genes, etc. • Technologies other than AAV <ul style="list-style-type: none"> ➢ Viral or non-viral vectors that can be applied to gene therapy • Technology to regulate expression levels <ul style="list-style-type: none"> ➢ Modified promoters, etc. ➢ having advantages over other technologies are desirable

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