

Needs & wants List (Nucleic acid drugs)

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|----------------------|---|
| 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">• Technologies to deliver nucleic acid drugs<ul style="list-style-type: none">➢ Improve pharmacokinetic parameters such as blood retention, tissue specificity/directivity and intracellular migration• Technologies to modify nucleic acid drug<ul style="list-style-type: none">➢ Technologies for new modifications and new skeleton of oligonucleic acid (those can improve efficacy, toxicity and kinetics)➢ Technology to enable oral administration of nucleic acid• Technology for expression-enhancing nucleic acids<ul style="list-style-type: none">➢ Unpublished targets are desirable |

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Needs & wants List (Gene therapy)

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| Diseases | Diseases amenable to treatment by in vivo gene therapy (excluding cancer viral therapy) |
| Object | <ul style="list-style-type: none">• Targets• Concepts• Technologies that can be used in drug discovery |
| Targets | Targets amenable to 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<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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