Anti-sense oligonucleotide profile

The following table represents example categories to consider in describing your current results/status and desired results at the end funding period.

Category	Parameters
Structure/Identity	Oligonucleotide sequence Backbone structure Other modifications Purity of test article achieved
In vitro activity	Treatment effect size on mRNA expression Treatment effect size on protein expression Dose response relationship for the above Selectivity/off-target effect to related target Selectivity /Broad panel
In vivo activity (such as target engagement/proximal downstream effect or efficacy) Indicate dose and route of administration	Concentrations of testing agent in the serum and target tissue Brain penetration Serum half-life, AUC or other pharmacokinetic properties Optimize Dose Selection and Schedule ED50, Minimal effective dose, and Optimal effective dose Treatment duration optimization Treatment window optimization Interactions with standard of care Selectivity/off-target effect in vivo
Safety	Tolerability Antibodies to product Tumorigenesis Assess host immune response Tissue Cross-Reactivity Local reactogenicity Cardiovascular, respiratory, renal and CNS safety pharmacology studies if any Genotoxicity
CMC	Process development status Analytical Assays for release testing with specifications Scale-up feasibility Formulation needs/development Stability Analysis Potency assays (in vitro/in vivo) Delivery devices
GLP analytical method development/validation	Potency assays, assays for purity, pharmacokinetics and pharmacodynamics etc
Interactions with regulatory agencies	Pre-IND
Patent protection?	