Gene therapy agent 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	Vector backbone, Transgene, Serotype, promoter, reagent purity achieved, etc.
In vitro activity	Treatment effect size on mRNA, protein expression, and dose response Selectivity/off-target effect to related target Selectivity to broader targets
Target tissue/cell biodistribution and expression/tropism	Short-term distribution Short-term expression (mRNA/Protein) Long-term distribution Long-term expression (mRNA/protein)
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 virus or gene product T-cell response Tumorigenesis
CMC	Master/Working Virus Bank Status 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-Pre-IND Pre-IND
Patent protection?	