This information should not be considered to represent advice or guidance on behalf of the U.S. Department of Health and Human Services or any agency or office thereof.

Example Target Product Profile (TPP) for a Cell and Gene Therapy

As explained in Creating a Target Product Profile for New Drug Products a TPP is a planning tool introduced by the FDA to streamline the drug development process. NIH SEED has developed the following sample TPP for a cell and gene therapy. It is intended to be illustrative and demonstrate potential content based on various stages of product development and a summary of the quality characteristics a drug product should possess. An actual TPP would require more detail and be based on thorough scientific, regulatory, and market research.

Sample Target Product Profile for a Cell and Gene Therapy (CGT)

Product Type: Gene Therapy	**Indication: Muscular Dystrophy**	
Attributes	**Minimal Acceptance Criteria**	**Preferred Acceptance Criteria**
Efficacy	Stabilization of disease progression	Significant improvement in muscle function
Safety & Tolerability	Comparable to standard care	Improved safety profile with minimal adverse events
Route of Administration	Intravenous infusion	Intravenous infusion
Dosage Form	Frozen viral vector solution	Lyophilized powder for reconstitution
Dosage Frequency	Single administration	Single administration
Stability & Shelf Life	Ultra-low freezer (-80 °C)	Stable at -20 °C

Preclinical Studies	
Vector Design & Testing	Optimize gene delivery vector for safety, targeting, and expression levels.
Efficacy in Disease Models	Demonstrate functional improvement in relevant animal models of muscular dystrophy.
Toxicology & Biodistribution	Assess off-target effects, potential for insertional mutagenesis, and vector biodistribution.

Quality	
Critical Quality Attributes (CQAs)	Identifiable physical, chemical, biological, and microbiological attributes within appropriate limits.
Sterility	For products that must be sterile, such as injectables.
Manufacturability	Considerations regarding manufacture, scalability, and yield of the production process.
Purity & Impurities Profile	Within appropriate limits for the host cell and manufacturing process.
Formulation	The presence or absence of an adjuvant or carrier and the type used can influence the gene therapy's safety, efficacy, and dosing regimen.
Immunogenicity	Potential for inducing an immune response within appropriate limits.
Potency	Biological activity within appropriate limits.
Cell Viability & Vector Titer	The number of viable cells or the titer of the viral vector within appropriate limits.
Cost of Goods (COGs)	Crucial for commercial viability and includes costs associated with raw materials, manufacturing, quality control, and packaging.

Clinical Trials	
Phase I/II	Early trials to assess safety, optimal dosing, and preliminary signs of efficacy in a small group of patients.
Phase III	Larger trials to confirm efficacy in improving or stabilizing muscle function and to monitor long-term safety and potential immune responses.

Market Analysis	
Patient Identification	Strategies for identifying and reaching the specific patient population may be small and geographically dispersed.
Pricing Model	Develop a pricing model that reflects the potentially transformative nature of the therapy, considering one-time administration.
Health Economics	Conduct health economic studies to demonstrate the value of potentially curative treatment in terms of long-term healthcare savings.

Regulatory Guidelines	
FDA/EMA	Follow guidelines for developing gene therapies, including long-term follow-up for safety and efficacy.
ICH	Compliance with ICH guidelines for gene therapies, including Q5A on viral safety.

Additional examples:

TPP for a Small Molecule Drug
TPP for a Biological Product
TPP for a Vaccine