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.

Target Product Profile Questionnaire

While FDA strongly advocates using a Target Product Profile (TPP), it does not mandate it. However, a well-organized TPP can streamline your interactions with FDA. A TPP can be especially critical at pre-Investigational New Drug meetings or pre-New Drug Application meetings because it helps FDA review staff focus on your goals and make sure previously agreed upon items have not changed throughout your development work. Note that TPP is a planning document. While verification of achieving milestones has value, TPPs are best used in planning the drug development process.
New innovators may find the following four-part questionnaire helpful in defining the requirements and the key attributes for drug products they are developing. There are several example TPPs (one of each type – small molecule, biologic, vaccine and cell and gene therapy).

Part 1: Target Identification and Candidate Development

Indication/Therapeutic Area
• What disease are you trying to treat? Are there current treatment options or are they poor or non-existent? What are the characteristics and claims of those treatment options or similar options? • What is the unmet need you are addressing? • Is the drug for treatment, prevention, or relief of symptoms? • Will the drug be used in conjunction with an existing primary therapy for a recognized disease or condition? • Is summary information available regarding completed or planned studies to support the target?
Product Class
• What is the modality (small molecule, peptide, siRNA, etc.)? • Is the product first-in-class therapy? • Is it single agent or coadministration as combination drug regimen?
Target Population
• What are the characteristics of those who will receive the intervention (e.g., age group, adults/adolescents/elderly)? Note any restrictions for the target population. • Are there any inclusion/exclusion criteria for immunocompromised and patients with comorbidities etc.? List any limitations of use. • Are the patients in the study representative of the primary at-risk population?

Clinical Efficacy
• Is the product/treatment superior and/or equivalent to same class or similar classes of compounds that have been approved? • How active (percentage) is the product against the target? • What Is the anticipated efficacy? • (A randomized, placebo-controlled study is the gold standard for efficacy.)
Clinical Study Endpoints
• What is clinically meaningful to patients and regulators? • What endpoints were used for current or similar options in the selected population? • If the primary endpoint requires a long time, are there interim endpoints or surrogate markers to provide evidence of benefit?
Clinical Pharmacology
• What is the clinical pharmacological effect of the drug with respect to mechanism of action, half-life, product elimination, absorption and tissue/organ/cell uptake, metabolites, concentration of drug in body fluids, degree of plasma binding, and passage across the blood-brain barrier?
Safety
• Is the safety and tolerability profile superior or non-inferior to levels of existing, comparable drugs? • What are the anticipated or accepted levels of adverse events? • Are there specific individuals who should not receive the product? • Has statistical analysis about benefit/risk ratio (efficacy/safety) been achieved in nonclinical/clinical trial?

Part 2: Drug Candidate Nonclinical Development and Efficacy

Efficacy in Nonclinical Studies, Animal Models
• Does the animal data indicate safe and effective use of the drug in humans? • Do the nonclinical studies meet the desired safety and efficacy endpoints? • How do the approved claims compare with same/similar products (efficacy and safety)? • How does this relate to a clinical condition? • If information is available, how do the claims of efficacy/safety compare with similar products, standard of care?
Pharmacology and Toxicology
• Do the Investigational New Drug (IND)-enabling nonclinical studies indicate the desired therapeutic effect, pharmacology (ADME), bioavailability and toxicology of the product? • Do the nonclinical studies inform the optimal dose (range finding), route, and frequency of dosing indicative of intended clinical formulation/usage? • Do the good laboratory practice (GLP) toxicology studies (single/multi-dose) in animal models provide definitive and statistical information regarding the drug products toxicity profile and maximum tolerated dose in comparison to a placebo? • Does the product induce off-target effects? • What is the pharmacological effect of the drug in nonclinical studies with respect to mechanism of action, half-life, product elimination, absorption and tissue/cell uptake, metabolites, etc.?

Part 3: Dosage and Formulation

Dosage Form
• What are the characteristics of the dosage form (e.g., liquid, solid, lyophilized powder, aerosolized)? • What is the color and size of the dosage form? Is the optimal dosage form therapeutically safe and effective in nonclinical/clinical studies? • Does the dosage form contain the intended amount of active drug? • How does the dosage form compare with similar products and standard of care?

Part 4: Additional Requirements to consider including in your TPP

Storage Requirements
• How long the product can be stored? • Are there specific constraints to maintain product quality and stability (e.g., do not freeze, sensitivity to light)? • Are there specific conditions that are critical for shipping, distribution, and maintaining the product critical quality attributes specifications and stability profile (e.g., sensitivity to shaking, secondary packaging to protect from light)? • Does the product have specific packaging requirements (e.g., ship under dry ice, ship with ice packs)?
Labeling
• Does the label specify usage indications? • Does the label on the container/package inform provide the dose strength/amount, dosage form, formulation, handling and storage conditions, and product expiry/or use by date? • Does the label include a product lot number and the manufacturer name? • Is the labeling in line with the regulatory requirements?
Product Registration and Prequalification
• What are the evidentiary requirements of FDA review and CMS coverage determination? • Has the innovator considered the pricing and reimbursement strategy needed in a complex regulatory environment to promote product registration and marketing? • How does the reimbursement approach compare with similar products standard of care?
Total Cost Per Patient
• Has the innovator conducted due diligence to create product differentiation and opportunity cost analysis to position, market, and price the product in relation to raw material and manufacturing cost of goods sold (COGS), other related marketed products, and medical need? • What is the breakeven point (i.e., what is the minimum cost per dose to recoup costs)?

Resources:

NIH SEED: Example TPP for a Small Molecule Drug
NIH SEED: Example TPP for a Biological Product
NIH SEED: Example TPP for a Vaccine
NIH SEED: Example TPP for a Cell and Gene Therapy