Example Target Product Profile (TPP)

A Target Product Profile (TPP) is a planning tool for therapeutic candidates based on FDA ***Guidance for Industry and Review Staff Target Product Profile — A Strategic Development Process Tool.***

The CBER Office of Cellular, Tissue and Gene Therapies (OCTGT) web page for industry education also has a Webinar on TPP **http://fda.yorkcast.com/webcast/Viewer/?peid=a53d0d5863244464b000249f1ddc9fd31d**

Below are example worksheets that define the minimal/ideal profile of the final marketed product and shows the ultimate goals of the proposed therapy development effort such as disease indication, patient population (with details such as symptomatic or pre-symptomatic patients for some genetic diseases), delivery mode, treatment duration, treatment regimen, and standards for clinical efficacy. The details of the TPP should be stage appropriate.

1. Example for ischemic stroke- revascularization

Product Targets	Minimum Acceptable Result	Ideal Results
Primary Product Indication	Emergency medicine for acute stroke patients immediately on hospital arrival	Emergency medicine for acute stroke patients in the community even before arrival to a hospital
Patient Population	Adults of all ages with moderate to severe stroke, with potential concurrent use with tPA	Adults of all ages with moderate to severe stroke, with potential concurrent use with tPA or replacement of tPA
Treatment Duration	Acute	Acute
Delivery Mode	IV	IV
Dosage Form	Solution in pre-filled syringes	Solution in autoinjectors
Regimen	Bolus	Bolus
Efficacy	20% or more favorable in comparison to placebo on minimal or no disability 30 days after treatment in patients using Modified Rankin Scale (score≤1) and NIHSS (score≤1). Exploratory endpoint: imaging evidence of revascularization	30% or more favorable in comparison to placebo on minimal or no disability 30 days after treatment using Modified Rankin Scale (score≤1) and NIHSS (score≤1). Exploratory endpoint: imaging evidence of revascularization
Risk/Side Effect	Devoid of symptomatic intracranial hemorrhage and significant mechanism related adverse effects	Devoid of any symptomatic intracranial hemorrhage and any mechanism related adverse effects
Therapeutic modality	Protein	

2. Example for pain associated with diabetic neuropathy:

Product Properties	**Minimum Acceptable Result**	**Ideal Results**
Primary Product Indication	Relief of pain symptoms in diabetic neuropathy	Relief of symptoms in neuropathic pain syndromes
Patient Population	Adults with diabetes who experience moderate to severe pain	Adults with diabetes who experience moderate to severe pain
Treatment Duration	Chronic	Chronic
Delivery Mode	Subcutaneous injections	Subcutaneous injections
Dosage Form	Prefilled vials with liquid	Prefilled vials with liquid
Regimen	Once every month	Once every 2 months
Efficacy	A 40% decrease in pain score in 30% of patients	A 70% decrease in pain score in 50% of patients
Risk/Side Effect	Devoid of local injection effect and clinically significant CNS side effect	Devoid of local injection effect and any CNS side effect
Therapeutic modality	Antibody	