**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](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

<table>
<thead>
<tr>
<th><strong>Product Targets</strong></th>
<th><strong>Minimum Acceptable Result</strong></th>
<th><strong>Ideal Results</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Primary Product Indication</strong></td>
<td>Emergency medicine for acute stroke patients immediately on hospital arrival</td>
<td>Emergency medicine for acute stroke patients in the community even before arrival to a hospital</td>
</tr>
<tr>
<td><strong>Patient Population</strong></td>
<td>Adults of all ages with moderate to severe stroke, with potential concurrent use with tPA</td>
<td>Adults of all ages with moderate to severe stroke, with potential concurrent use with tPA or replacement of tPA</td>
</tr>
<tr>
<td><strong>Treatment Duration</strong></td>
<td>Acute</td>
<td>Acute</td>
</tr>
<tr>
<td><strong>Delivery Mode</strong></td>
<td>IV</td>
<td>IV</td>
</tr>
<tr>
<td><strong>Dosage Form</strong></td>
<td>Solution in pre-filled syringes</td>
<td>Solution in autoinjectors</td>
</tr>
<tr>
<td><strong>Regimen</strong></td>
<td>Bolus</td>
<td>Bolus</td>
</tr>
<tr>
<td><strong>Efficacy</strong></td>
<td>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</td>
<td>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</td>
</tr>
<tr>
<td><strong>Risk/Side Effect</strong></td>
<td>Devoid of symptomatic intracranial hemorrhage and significant mechanism related adverse effects</td>
<td>Devoid of any symptomatic intracranial hemorrhage and any mechanism related adverse effects</td>
</tr>
<tr>
<td><strong>Therapeutic modality</strong></td>
<td>Protein</td>
<td></td>
</tr>
</tbody>
</table>
2. Example for pain associated with diabetic neuropathy:

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