

# Incorporating Patient Input into the Target Product Profile (TPP): A Patient Focused, Standardized Guidance Framework

**Authors:** Jean-Noel Talabardon<sup>1</sup>; Janet Church<sup>2</sup>; Masanori Okuse<sup>3</sup>; Michaela Dinboeck<sup>1</sup>; Sheila Dickinson<sup>1</sup>; Simon Messner<sup>1</sup>; Michael Boisclair<sup>4</sup>; Jean-Pierre Malkowski<sup>1</sup>

**Affiliations:** <sup>1</sup>Novartis Pharma AG, Basel, Switzerland; <sup>2</sup>The Sjögren's Foundation, Reston, VA, USA; <sup>3</sup>Japanese Association for Psoriatic Disease, Yokohama, Japan; <sup>4</sup>Novartis Biomedical Research, Cambridge, MA, USA

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## Introduction

### What is a TPP:

A Target Product Profile (TPP) is a strategic document which summarizes characteristics necessary for a medicine to meet patients' needs and priorities, receive regulatory approval and reimbursement, and differ from existing treatments.<sup>1,2</sup>

### Why it matters:

Patients, as experts in their experience of their own disease, bring invaluable perspectives that cannot be obtained by other means. This input improves relevance, endpoints, trust, and eventual adoption.<sup>3</sup>

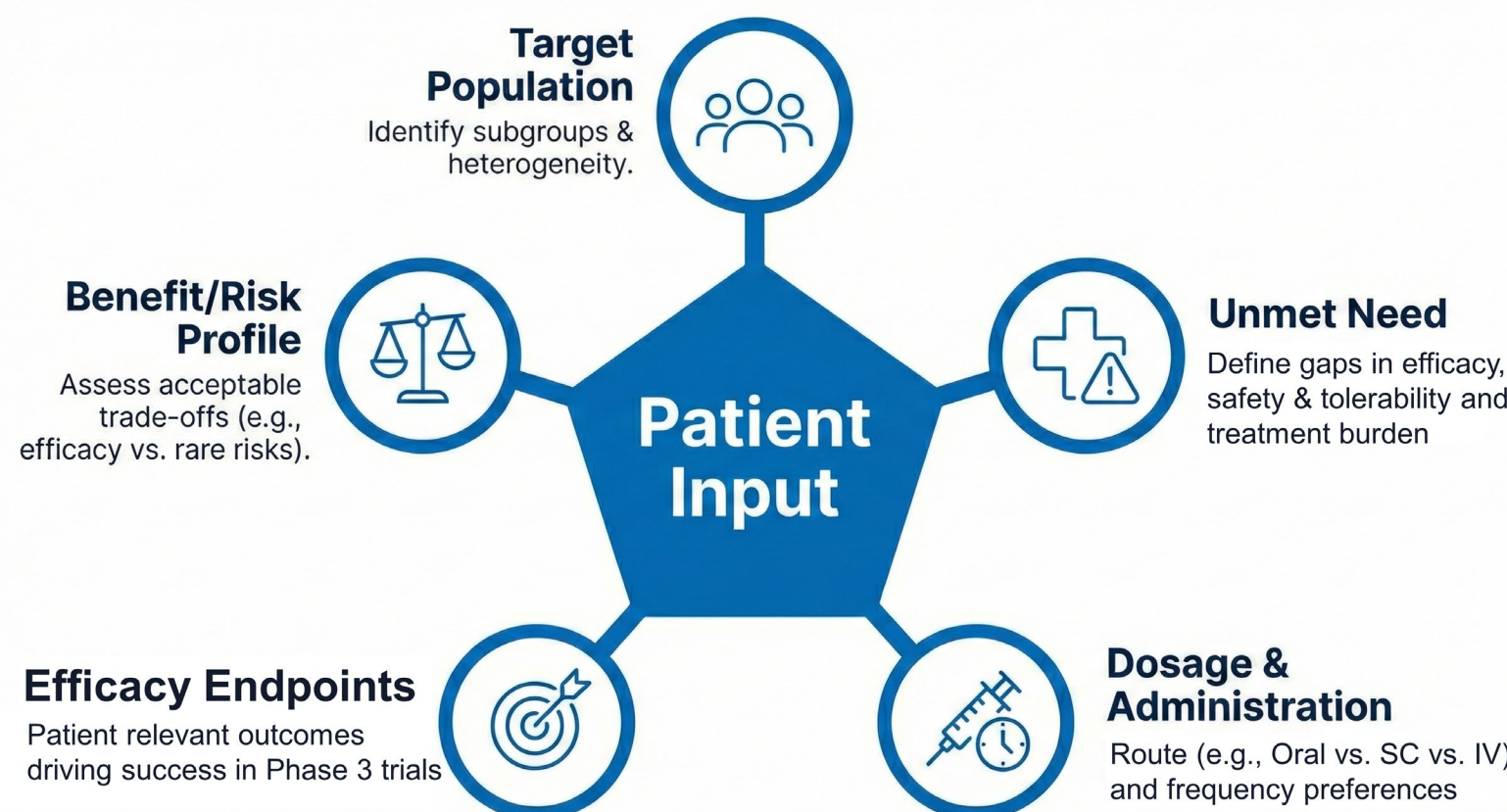
## Objective and Methodology

To present a systematic, patient-focused guidance framework for developing a standardized TPP, co-reviewed and validated with the patient community, and applicable from pre-clinical stages onward.

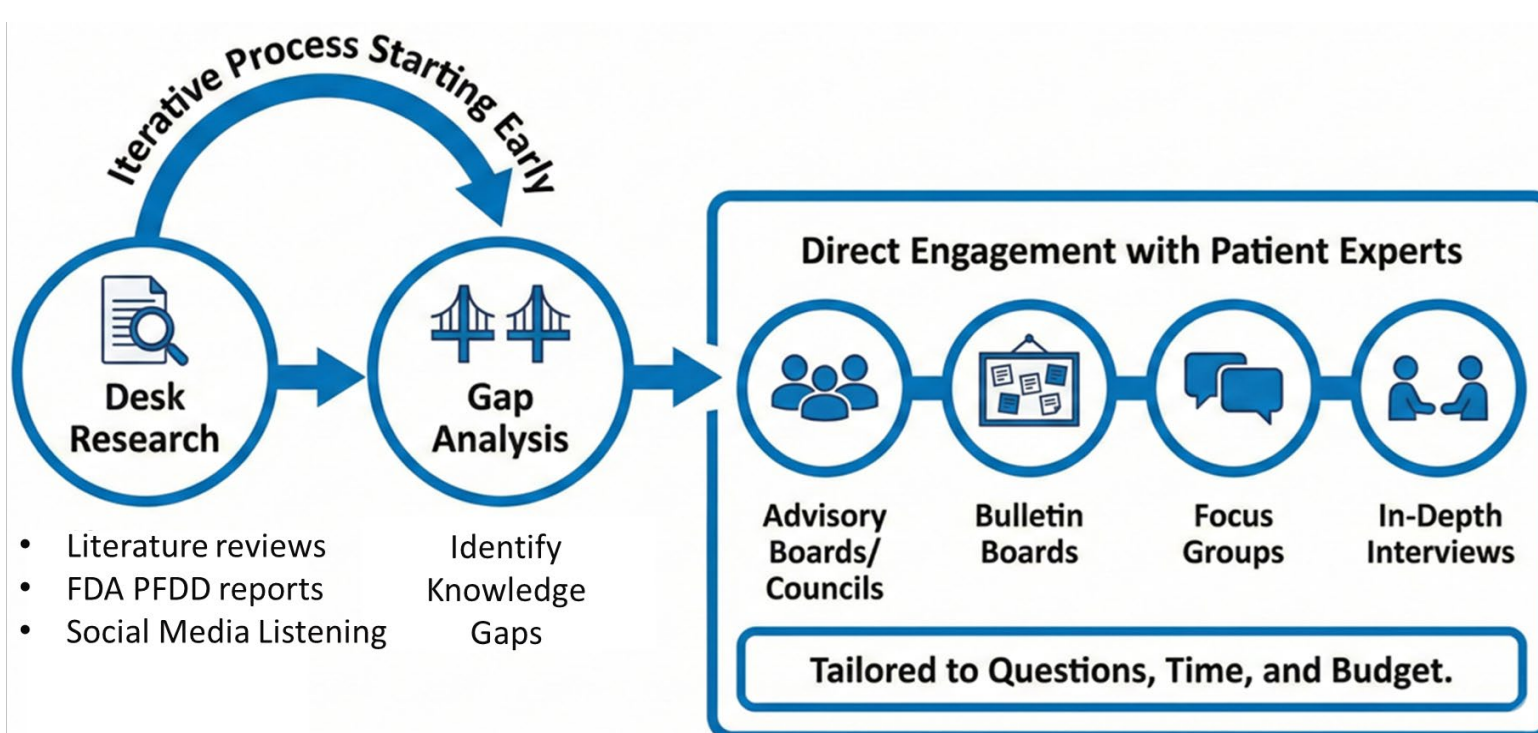
## Results

### Systematic guidance framework

#### 1. Five focus areas for patient input



#### 2. Methods for collecting patient input<sup>4</sup>

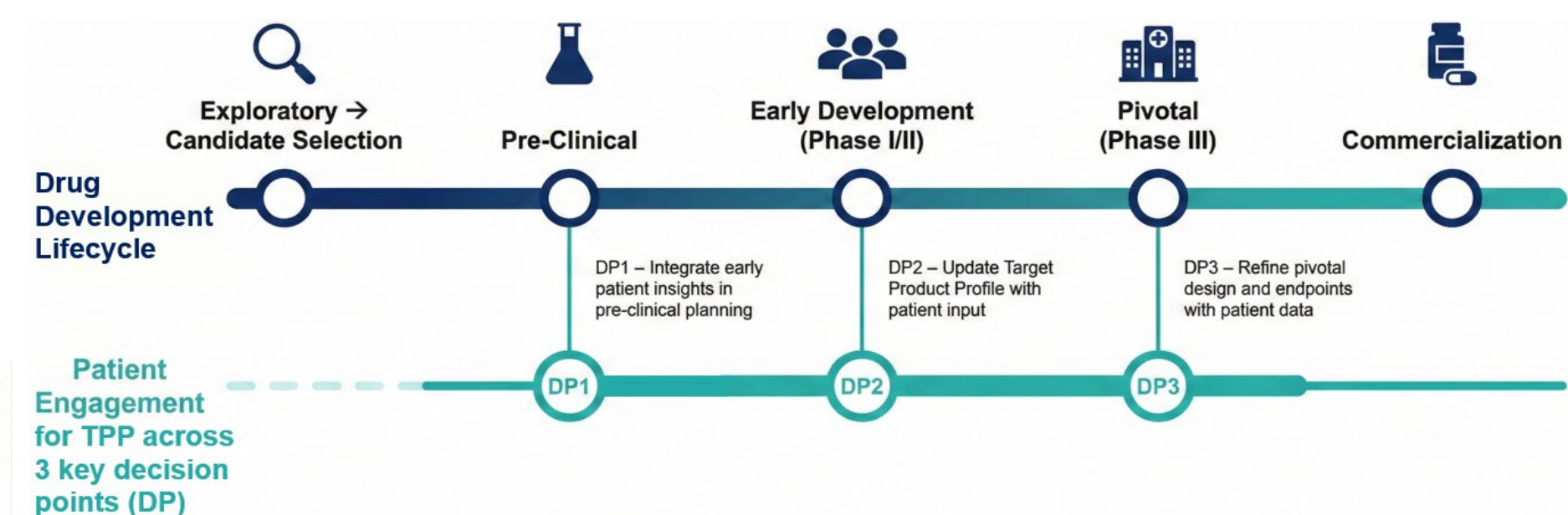


### 3. Enablers for high quality patient input

- ✓ **Use clear, accessible language** (lay terms, simple visuals, avoid jargon)
- ✓ **Show respect** (recognise patients as whole persons not subjects)
- ✓ **Engage patient experts** (with disease knowledge and representing community voice)
- ✓ **Provide context** (severity, stages, treatment landscape)
- ✓ If applicable **explain novel mode of action** (keep it simple and visual)
- ✓ If applicable, provide **context relevant to discussion of benefit-risk trade-offs** (information on the alternative treatment options etc.)

### Suggested timepoints for patient input to TPPs

Figure 1: Development and patient engagement for TPP timeline integration



### Case examples

Table 1: Case examples of patient input shaping the TPP across key focus areas

Focus Area	Methods	Indication	Patient Input	Impact on TPP
Target population	Patient Council Review <i>(Novartis internal)</i>	Food allergy	Age-group is the key defining factor, more important than disease type.	More focused, age-segmented TPPs
Unmet need	Patient Advisory Board <i>(Novartis internal)</i>	Dry eye disease	Preference for convenient treatments; twice-daily dosing; less artificial tears; ocular surface pain relief.	Defined key product characteristics for the TPP.
Dosage/Route	Randomized, open-label crossover study with patient preference questionnaire	DLBCL/FL (rituximab)	Strong preference for subcutaneous over IV (shorter time and comfort).	Can inform route assumptions for future therapies.
Endpoints	International iterative process with CML advocates network	CML	EORTC QLQ-CML24: 24 items on symptoms and quality of life.	Can guide endpoint selection in TPPs/trials.
Benefit–Risk	Online discrete-choice experiment based on patient interviews <sup>5</sup>	Alopecia areata	≥50% scalp regrowth is pivotal; adults vs. adolescents differ in risk concerns.	Can quantify acceptable benefit-risk trade-offs for TPP claims

The first two examples (in blue text) illustrate the active engagement by Novartis with patient experts; the following three (in black text) show how information available via desk research can inform a TPP.

**Abbreviations** – IV: Intravenous; SC: Subcutaneous; FDA: Food and Drug Administration; PFDD: Patient-Focused Drug Development; DLBCL: Diffuse Large B-Cell Lymphoma; FL: Follicular Lymphoma; EORTC QLQ-CML24: European Organisation for Research and Treatment of Cancer Quality of Life Questionnaire-Chronic Myeloid Leukaemia 24-item module; CML: Chronic Myeloid Leukaemia; EMA: European Medicines Agency; PMDA: Pharmaceuticals and Medical Devices Agency; AI: Artificial Intelligence

## Key takeaways

- **First systematic framework:** This represents the first systematic guidance for developing patient-focused, standardized TPPs validated with the patient community.
- **Early integration essential:** Patient perspectives must be incorporated systematically starting as early as possible in drug development across the five critical focus areas including target population, unmet need, dosage/route of administration, efficacy endpoints, and benefit/risk trade-offs.
- **Efficacy endpoints are most important:** if TPPs include patient-relevant endpoints accepted by regulators in pivotal trials, drugs can launch with patient-relevant label claims.<sup>6</sup>
- **Adaptation needs:** Implementation requires adequate human and financial resources, plus translation between technical TPPs and patient-friendly versions. Organizations should leverage existing patient experience data before direct engagement.
- **Broader impact:**
  - **Enhanced recruitment** and retention of patients participating in clinical trials, health equity focus, and treatments aligned with patient needs
  - Improved alignment with FDA, EMA, and PMDA expectations and strengthened evidence base for **regulatory approval and access**
  - **Patient empowerment:** patients gain a stronger voice, treatments better matched to their lived experience, and greater understanding and influence over drug development priorities.
- **Future developments** like Core Outcome Sets and Patient Experience Data Dossiers will optimize this framework, providing value to all stakeholders. Natural language processing (AI) methods could enhance analysis of qualitative patient data.<sup>6</sup>
- **Call to action for patient organizations** to communicate their needs and preferences regarding the five focus areas, in order to maximize their influence on future TPPs.

### ❖ Future Vision

- The systematic integration of patient perspectives into TPPs has the potential to:
  - Support implementation of a patient-focused drug development approach
  - Provide value to all stakeholders
  - Enhance recruitment and retention of patients participating in clinical trials
  - Lead to development activities and hence drug labels that are more likely to address patient-relevant endpoints

## Selected References

(To read the full list of references, click on the link for full publication at the bottom of this poster)

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### Disclosures

**Conflicts of Interest:** JNT, JPM, MB, MD, SD, and SM are employees of Novartis. JEC and MO report that their affiliated organizations have received support from Novartis, but that they did not receive payment for their contribution and participation in this publication.

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### CONTACT INFORMATION

Corresponding Author:  
Jean-Noel Talabardon,  
Novartis Pharma AG, Asklepios Building - 5th Floor, Novartis Campus, 4056, Basel, Switzerland  
Email: [jean-noel.talabardon@novartis.com](mailto:jean-noel.talabardon@novartis.com)