Patient Engagement open forum

Into Reality

How to engage patients in the early stages

This session will start at
9:00 EST/ 15:00 CEST

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

PATIENT FOCUSED MEDICINES DEVELOPMENT

EUPATI
European Patients' Academy on Therapeutic Innovation

REC **Disclaimer:** This meeting is going to be recorded.

Welcome to the Patient Engagement Open Forum virtual session

Patient Engagement open forum Into Reality

Patient Engagement Open Forum is a series of virtual events (in 2020) where we will work together, in a multi-stakeholder context, **to turn patient engagement from an aspiration into reality.**

The Forum aims to provide **a holistic perspective** of patient engagement, the **landscape and actors**, and **foster collaboration** and **co-creation** while **breaking down fragmentation** that are often present in patient engagement work.

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Agenda

Welcome and introductions

Where do we come from? Why how-to guides?

What is this how-to guide?

a. Preparation and understanding condition profile

Break

b. Research methodology

c. From Target Product Profile to Target Value Profile

Next steps and close

Before we get started, we ask you to

- Be present and engaged
- All microphones will be in mute
- Please provide your questions, comments & feedback in the Q&A function
- This session will be recorded
- Let's make this workshop interesting together!

Welcome!

We’re so happy to see you here!

Speakers today

Nick Hicks
Commutateur Advocacy Communications

Carole Scrafton
CEO / Co-Founder Patient Advocacy Organisation & Community Support Online Network

Dr Oleksandr Gorbenko, MD, PhD
Global Patient Centricity Director IPSEN

Dr. Natasha Ratcliffe (WG co-lead)
Research Involvement Manager Parkinson's UK

Oana Bernard-Poenaru
Patient Officer - Clinical Research – R&D, Servier

Merlin Williams
Senior Consultant, Executive Insight, Healthcare Consultants

Chi Pakarinen
Programme Manager The Synergist

David Feldman
Medical Project Director National Kidney Foundation

How about you?

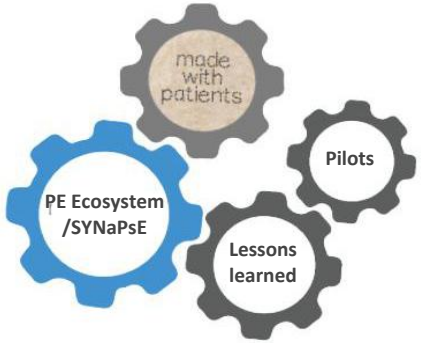
- Which stakeholder group do you represent?
- Did you attend our session last year?
- Do you have experience of doing patient engagement/
  - or if you are a patient: being involved as a patient in the early stages?
- Are you currently using any patient engagement methodology or guidance?

Now that we know each other a bit more,
let's continue!

PFMD - a global, multi-stakeholder collaboration

PFMD's systematic approach to co-creation

Landscape & Need Analysis	Co-Creation **WITH** Patients for patients	PE methodology and ecosystem development	Patient Engagement Management (PEM) [2018 focus]



Communication and outreach activities

The Patient Engagement Quality Guidance and 7 PE Quality Criteria

A multi-stakeholder collaboration to create **standards for good patient engagement** and support partnership setting between patient community and other stakeholders.

- **2016-2018**
- **Outcomes:**
  - Patient Engagement Quality Guidance
  - Book of Good Practices
  - Set of Do's and Don'ts
- **Now:** detailed how-to guides

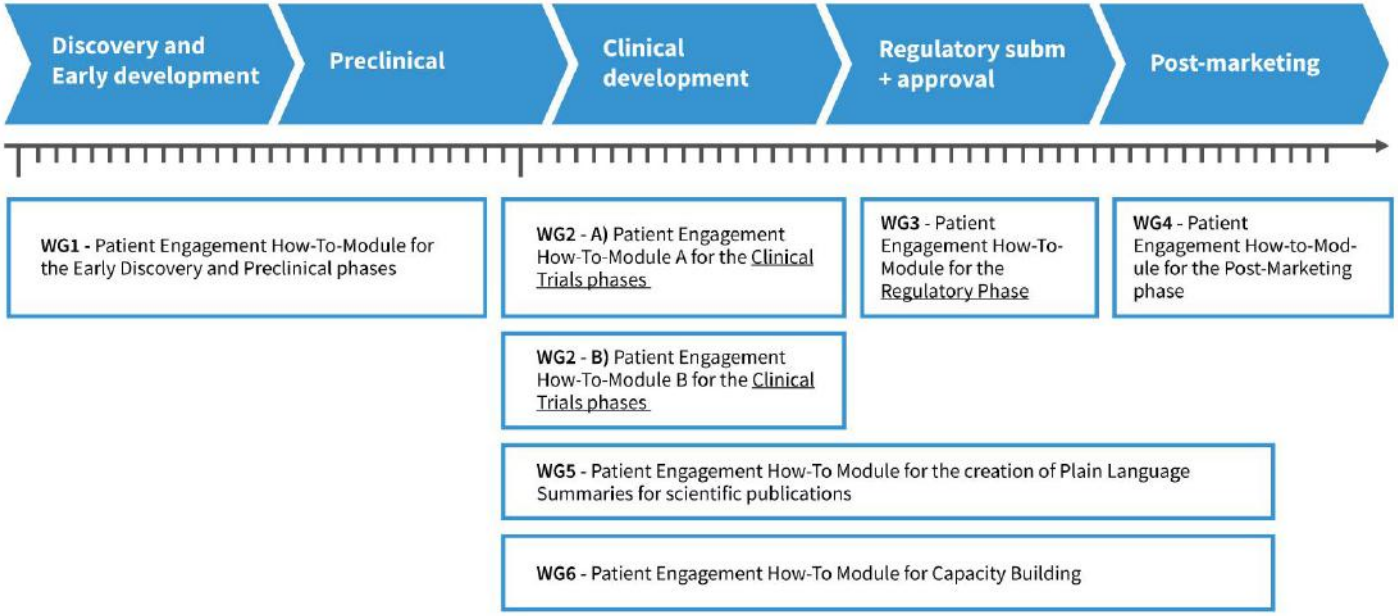
A network of committed and active contributors to the Working Groups

103 Participants, representing
58 Organisations, in
* **6** Working Groups

40 Industry representatives
18 Patient/ patient organisation representatives
8 Consultancy representatives
12 Patient experts/ advocates
4 CRO/ Service providers to pharma industry-representatives
3 Publishers
1 Academic researchers
2 Research Institute

2 Independent experts with various related expertise
2 Medical Communications Agency
2 Research Hospital representative
1 National public and patient involvement organisation
1 Young Patients Advisory Network representative
1 HTA
2 Regulator
2 Clinical Researchers
2 Public-Private Partnership

Detailed how-to's at every step of the way



PE Quality Guidance (as the "backbone" of all how-to modules)

Working Group 1

HOW-TO engage patients in early discovery and preclinical phases

Objective of this group

To co-create a detailed and comprehensive how-to guide with additional resources and tools that helps stakeholders to engage patients in the early phases.

Progress so far

This multi-stakeholder has created a sequential approach for involving patients as partners from insight generation to evaluating research methodologies in the early discovery and preclinical phases, and hence increasing the impactfulness of PE in the early stages.

First iteration was shared in the PEOF2019 and the close to final version now in the PEOF2020. The draft will go out for public consultation during Q3 and Q4 of 2020

Working Group 1 Milestones

Q4/ 2020 — Finalisation for launch

Q3/ 2020 — External reviews and public consultation begin

Q2/ 2020 — Drafting, testing & reviews within group

Q1/ 2020 — How-to structure agreed, drafting begins

2019 — Content Definition

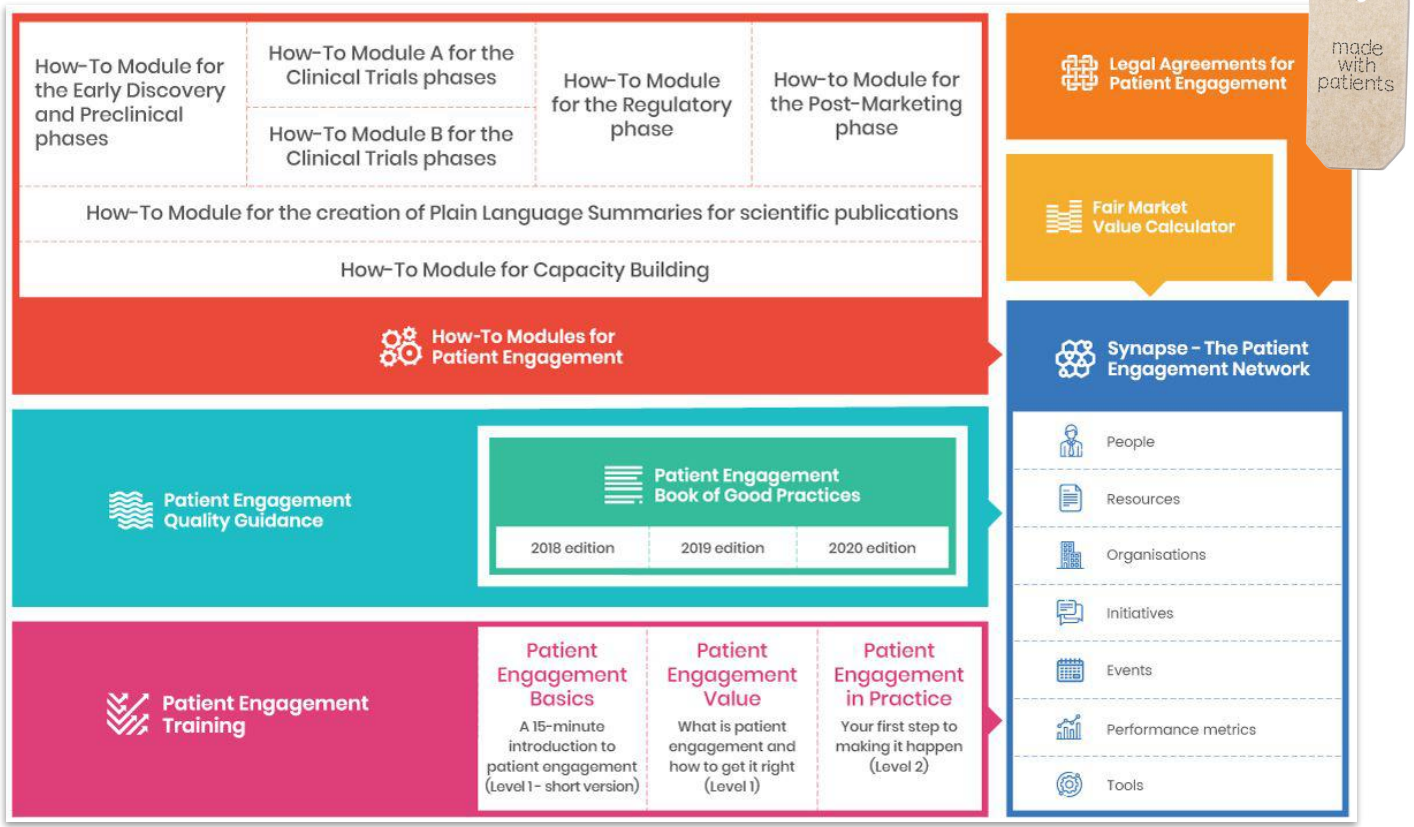
CORE TEAM

Benjamin Missbach	Ludwig Boltzmann Gesellschaft
Carole Scrafton	Patient advocate/expert/Fibro Flutters
Claire Nolan	Charities Research Involvement Group
David Feldman	National Kidney Foundation
Dawn Richards	Patient advocate/CAPA, CTO
Grace Fox Manoj Lalu	The Ottawa Hospital
Merlin Williams	Executive Insight, Healthcare Consultants
Natasha Ratcliffe	Parkinson's UK
Nick Hicks	Commutateur Advocacy Communication
Oana Bernard-Poenaru	Servier
Oleksandr Gorbenko	Ipsen Pharma
Vivian Larsen	Takeda

REVIEWERS (+ more)

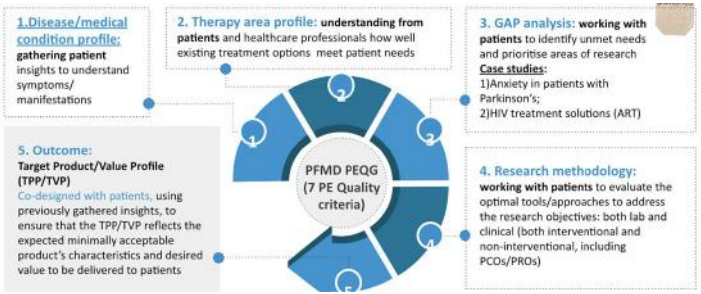
Abbe Steel	Healthivibe
Anne Charlotte Fauvel	EATRIS
Deborah Bertorello	Multi-Act
Ganive Bhinder	Better Pharma Care Coalition
Jennifer Preston	eYPAGNet
Katherine Deane	University of East Anglia
Kelli Collins	National Kidney Foundation
Neil Bertelsen	HTAi
Paola Zaratin	Multi-Act
Rie Kunisada	Takeda
Ursula Davis	El8 Consulting
Wendy Costello	Patient advocate/ expert/iCAN

Patient Engagement Management Suite



Accessible at www.pemsuite.org

Evolution of the model with the feedback gathered



made with patients

Deep dive into the sections of this how-to

Presentation 10-15’	Questions & Answers 10’	Polling 5’
Use Q&A to ask questions and comment during the presentations	Q & A	Use your computer or phone to answer poll

Preparation for collaboration &
Understanding the condition

Carole Scrafton
CEO / Co-Founder Patient Advocacy Organisation & Community Support Online Network

Dr Oleksandr Gorbenko, MD, PhD
Global Patient Centricity Director IPSEN

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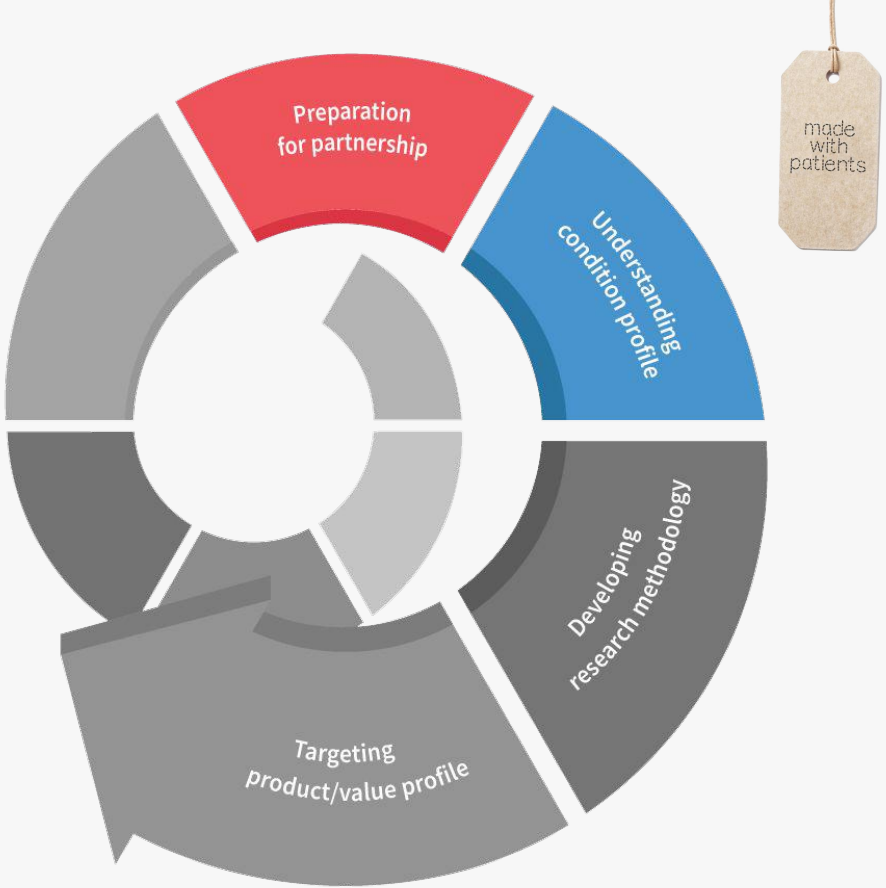
Preparation for partnership and understanding the condition profile



The importance - THE WHY

Discover the purpose of your study

Patients are the best equipped to understand the condition

Establish relationships with patients / patient organisations

Engage to **discover patient viewpoints** of their condition and their ability to contribute

Patients get to work with researchers

Opportunity **to educate both the patients, and industry about early discovery and preclinical research** and the importance of it

Path to gathering patient input

Define collaboration goals	Identify potential patients	Profile potential patients	Select & invite patients	Educate patients on Preclinical Research	Develop questions	Patients ready	Initial Forum (Patient-Researcher Meeting)

▪ Patient Organization
▪ Industry & Academia
	▪ Patient Organization	▪ Patient Organization	▪ Patient Organization
			▪ Industry & Academia
				▪ Industry & Academia	▪ Patients
					▪ Patient Organization
					▪ Industry & Academia
							▪ Patients
							▪ Industry & Academia
							  ▫ Preclinical Research Team
							  ▫ Clinical development
							  ▫ Marketing?
							▪ Patient Organization
							▪ Moderator

	Patient Organization database ▪ Known patients ▪ Email, social media	▪ Survey		▪ Webinar ▪ Other information			▪ Patient Surveys ▪ Periodic Meetings ▪ Patient Advisory Board Meetings

Condition profiling

To find out what the **unmet needs** are and what's most important to the patient community:

- Gather as much information about the **patients with the condition**
- Discovere the **'patient' experience** and perspective of what it is like to live with the condition

Therapeutic area profiling

To find out **if the existing treatment options meet patient needs**:

- Gather information about the condition itself
- Asking patients and HCP for **their insights and understanding of the condition**
- Seek to understand which therapy and treatments treat which parts of the condition.
  - What therapies exist for the disease?
  - Are there known side effects?
  - What types of patients do you need?
  - What other research is currently being carried out for your chosen condition area?

Why they are important

- Help you wean out the **essential information** required for your study
- To establish **key relationships** with patients, and other relevant stakeholders
- **Discovering unmet needs of patients**
- Lay down the **foundations** of your study
- **Educate to all parties** what is essential, and why these processes are important

Creating a win-win scenario

As scientists, or researchers this should be at the forefront of your trial design and this can be achieved if you

- Incorporate the views of all stakeholders by working collaboratively
- Share knowledge with each other.
- Engage as a team to prevent bottlenecks from the start.

Gap analysis

Existing care options:

- Standard of Care (SoC) as per actual guidelines and protocols
- Established best practices for the dedicated medical condition(s)

Desired care options, as per:

- Unmet medical needs
- Expectations
- Preferences
- Value to be delivered through innovation (to be presented under TPP/TVP sections)

Q&A for Preparations and Understanding the condition section

What do you think about the section?

Answer the poll in menti.com - with the code: 481 781

- Is this section comprehensive for the purpose?
- Would this section be useful for you?
- What would you add to make it more useful?

Coffee break

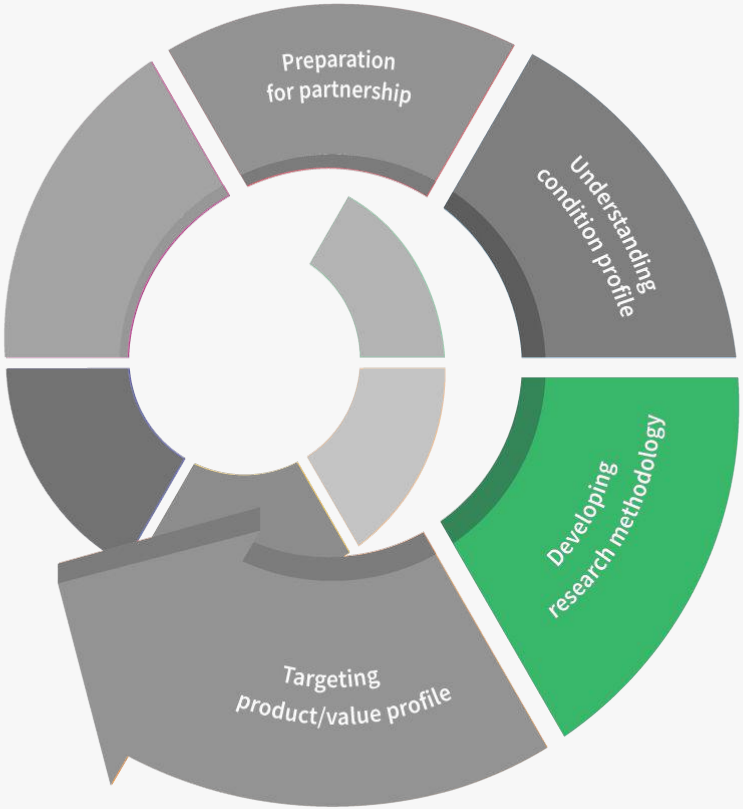
10 minutes

Creating a research methodology

Oana Bernard-Poenaru
Patient Officer - Clinical Research – R&D, Servier

Nick Hicks
Commutateur Advocacy Communications

Creating a research methodology



Creating a research methodology with patients

Objective

Identifying the most suitable tool / approaches to capture and translate patient insight into early stage R and D activities

Benefits

- ✓ Shape the way research is conducted (methodological)
- ✓ Lead to early adaptations of research towards a more patient-focused design of studies

Fundamental steps to consider

Research priority setting (RPS) ensuring that research priorities align with the patient priorities

- Remember something of scientific interest or what HCPs think is important may not be important for disease sufferers eg Burn Management

Powerful questions to ask at this time

- When is the best time for RPS?
- What Research aspects/topics can be discussed?
- What methods are best?
- How to facilitate such meetings?
- Who needs to be involved in the RPS?

Before the patient engagement starts

Understand capacity building needs of both researchers and patients

- What training is needed on both sides for an efficient dialogue?
- Draft research plans ready in patient friendly language?

Appoint steering group (SG) for project management

- Focus on getting right mix of people and align/manage various expectations

Kick start the project

- What's the best way?

Create a timeline of key milestones (Ways of working)

- Identify how SG will meet
- How will feedback be given, received and used?

Formats of engagement

Format	Often used for:
Virtual engagement	Allow more participation, less logistical planning, in-depth meetings or introduction to topics - versatile usage
Patient steering committees	Working with industry
Focus groups	For gathering patient insights on general topics
Round tables	For gathering patient insights on specific topics
Online surveys	For questions that require a high number of respondents to validate
Patient expert panels	For specific topics
Written patient feedback	After meetings or interactions. Via email, mail or live after the session.
One to one patient interviews	(often by phone)
Webinars and webinar feedback	For disseminating information

Choosing the best suitable format for your engagement

Depends on your

- Objectives and Appropriateness
- Disease
- Budget and Timelines (and maturity of the research project in the development cycle)
- What's worked before
- Compliance

Stakeholder representations

Creating Industry / Patient Research team

- Understand who are the key players

What is the best way to ensure a diverse patient group?

- Patient groups give more generalised insight
- Patient advocates give raw unfiltered insight
- What type of patient advocate
  - Disease specific
  - Specialist in PRO
  - Publications

All have a role to play at different times and with different methods

Take home messages

Start as early as realistically possible

- Prepare internally (on both sides) before you go external

Be very clear at the start what you need, what you want the other party patient community to do and what you need or what you won't commit to

- Be 100% transparent

for pharma, understand where the Patient Group / Advocate / Patient is with respect to the type / process of interaction required and assess also internal skills needed for this collaboration

- Identify capacity building needs

Patient groups now working to two agendas; their own and COVID 19

- Be ready to help navigate when needed

Get a diverse mix of patients reflecting the disease spectrum

- Who is best to give specific types of insight and when

Be realistic on both sides on what can be achieved

- Build in reality checks to measure progress

Q&A for Developing Research Methodology section

What do you think about the section?

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- What would you add to make it more useful?

From Target Product Profile to Target Value Profile

Dr Oleksandr Gorbenko, MD, PhD
Global Patient Centricity Director IPSEN

Merlin Williams
Senior Consultant, Executive Insight, Healthcare Consultants

Target Product Profile (TPP) and Target Value Profile (TVP)

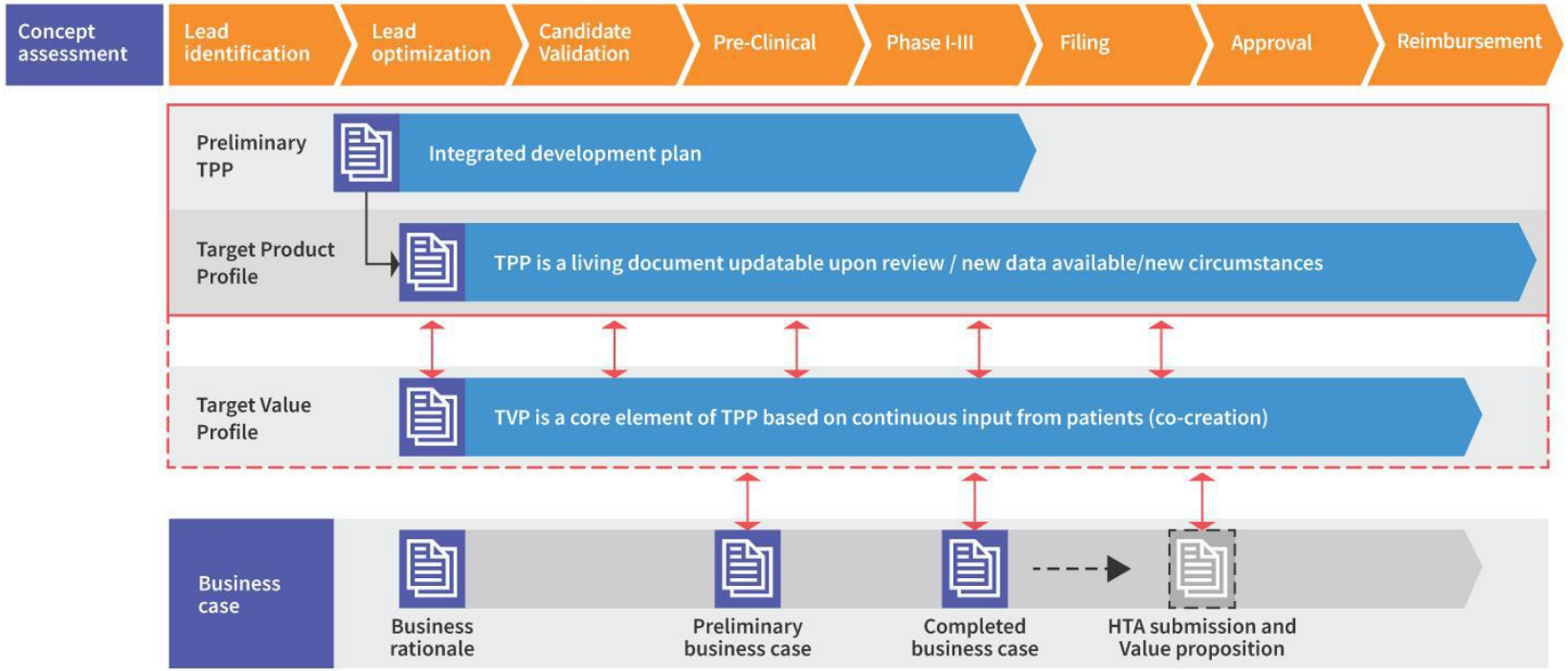
TVP/ TPP - Definitions of Target Value and Target Product profile

A **target value profile (TVP)** is an essential part of early drug development. It helps companies and researchers plan the development of a new medicine.

The TVP is a consolidated set of “expected and minimally acceptable characteristics” of a medicinal asset, biological product, or medical device, which are valuable and meaningful for patients by addressing areas of remaining unmet needs.

Alongside business rationale, public health factors and other elements for decision making, the TVP informs the **target product profile (TPP)** – an updatable guidance for the pharma industry/ drug developers with targeted characteristics of a potential asset/product

Timeframes


Concept assessment
Lead identification
Lead optimization
Candidate Validation
Pre-Clinical
Phase I-III
Filing
Approval
Reimbursement
Preliminary TPP
Integrated development plan
Target Product Profile
TPP is a living document updatable upon review / new data available/new circumstances
Target Value Profile
TVP is a core element of TPP based on continuous input from patients (co-creation)
Business case
Business rationale
Preliminary business case
Completed business case
HTA submission and Value proposition

Key elements

TPP element	What does it mean for developers (TPP)	What does it mean for patients (Value)
Indication		
Target population		
Efficacy and Effectiveness **Resistance** (for antimicrobial agents and some other medicines) **Specificity and Sensitivity** (for diagnostics)		
Safety profile		
Tolerability profile		
Clinical pharmacology		
Dosage and administration (posology)		
Storage conditions		
Business rationale (business case – may/may not be a part of TPP)		
Value proposition/value positioning		

Although the TVP has the same key elements as the TPP, with minimally acceptable characteristics in each element, their interpretation differs because they address what patients valued most.

Guidance contains the list of questions to be discussed under TVP

Example I: Tolerability under TPP and TVP

TPP element	What does it mean for developers (TPP)	What does it mean for patients (TVP)
Tolerability profile	• Non-inferior/superior tolerability profile in comparison of Standard of Care (reported as PRO); • % of potential users adapting to tolerability issues within one round of use; • % of discontinuation due to tolerability issues; • No irreversible tolerability issue (issue that does not resolve after discontinuation of drug);	• *What are the expected tolerability issues of the proposed treatment (please, note: tolerability issues may be reported as relevant PRO measurements in clinical trials; at the stage of TVP development it's important to consider patient expectations from tolerability profile)?* • *What kind of PRO/PCO measurements and tools should be used reflecting tolerability profile in the forthcoming studies?* • *% of study participants who have accepted/adapted to possible tolerability issues?* • *% of study participants who have discontinued due to tolerability issues?* • *Any expected irreversible tolerability issues?*

Example II: Dosage and administration under TPP and TVP

TPP element	What does it mean for developers (TPP)	What does it mean for patients (TVP)
Dosage and administration (posology)	• Formulation/formulations; • Types of administration/delivery; • Injection site/sites; • Injection volume; • Dosing frequency; • Number of pills per dose; • Dosing timing; • Dosing with relation to food; • Dosing adjustments (see the factors above); • Pill size; • Coformularity - ability to be co-formulated into fixed dose combinations and/or single tablet/injection regimens; • Other posology aspects for alternative formulations;	• *What are the most/least desirable formulations for this treatment?* • *What are the most/least desirable ways of delivery for this treatment?* • *Any changes in terms of formulations/ways of delivery vs existing SoC?* • *Desirable/acceptable injection sites?* • *Desirable/acceptable dosing frequency?* • *Desirable/acceptable number of pills per dose?* • *Desirable/acceptable dosing time?* • *Relation to food and drinks?* • *Relation to daily activities: physical, mental, sexual, working/daily routine, childbearing/breastfeeding?* • *Dependence from HCPs/clinics or caregivers in terms of administration/delivery;*

Q&A for Target Value / Product Profile section

What do you think about the section?

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- Would this section be useful for you?
- What would you add to make it more useful?

Next: finalising this work

Public consultation end of summer/ early autumn

Would you like to pilot the guidance?

Ending poll: in Menti.com - code: 726 661 - will be open until tomorrow

- Which of the sections is the most important to you?
- How would you **use** this guidance? (Choose from options)
- How likely would you use this **how-to guidance**? (scale)
- How likely would you **recommend** this how-to guidance? (scale)

Thank you for joining us today!

For further information about the work,
please send us an email to

support@pfmd.org