

# How to engage patients in the early stages

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



Disclaimer: This meeting is going to be recorded.

# Welcome to the Patient Engagement Open Forum virtual session

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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.

# Agenda

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**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

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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

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**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?

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- 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

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# PFMD's systematic approach to co-creation

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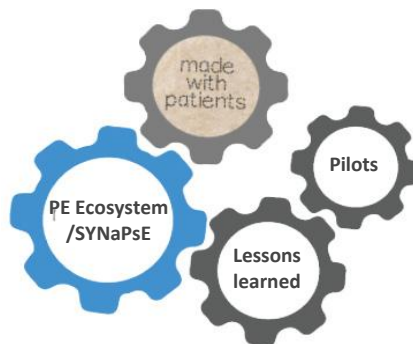
## Landscape & Need Analysis



## 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



**WG1** - Patient Engagement How-To-Module for the Early Discovery and Preclinical phases

**WG2 - A)** Patient Engagement How-To-Module A for the Clinical Trials phases

**WG3** - Patient Engagement How-To-Module for the Regulatory Phase

**WG4** - Patient Engagement How-to-Module for the Post-Marketing phase

**WG2 - B)** Patient Engagement How-To-Module B for the Clinical Trials phases

**WG5** - Patient Engagement How-To Module for the creation of Plain Language Summaries for scientific publications

**WG6** - Patient Engagement How-To Module for Capacity Building

**PE Quality Guidance (as the “backbone” of all how-to modules)**



### 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



### CORE TEAM

Benjamin Missbach  
Carole Scrafton  
Claire Nolan  
David Feldman  
Dawn Richards  
Grace Fox  
Manoj Lalu  
Merlin Williams  
Natasha Ratcliffe  
Nick Hicks  
Oana Bernard-Poenaru  
Oleksandr Gorbenko  
Vivian Larsen

### REVIEWERS (+ more)

Abbe Steel  
Anne Charlotte Fauvel  
Deborah Bertorello  
Ganive Bhinder  
Jennifer Preston  
Katherine Deane  
Kelli Collins  
Neil Bertelsen  
Paola Zarin  
Rie Kunisada  
Ursula Davis  
Wendy Costello

Ludwig Boltzmann Gesellschaft  
Patient advocate/expert/Fibro Flutters  
Charities Research Involvement Group  
National Kidney Foundation  
Patient advocate/CAPA, CTO

The Ottawa Hospital

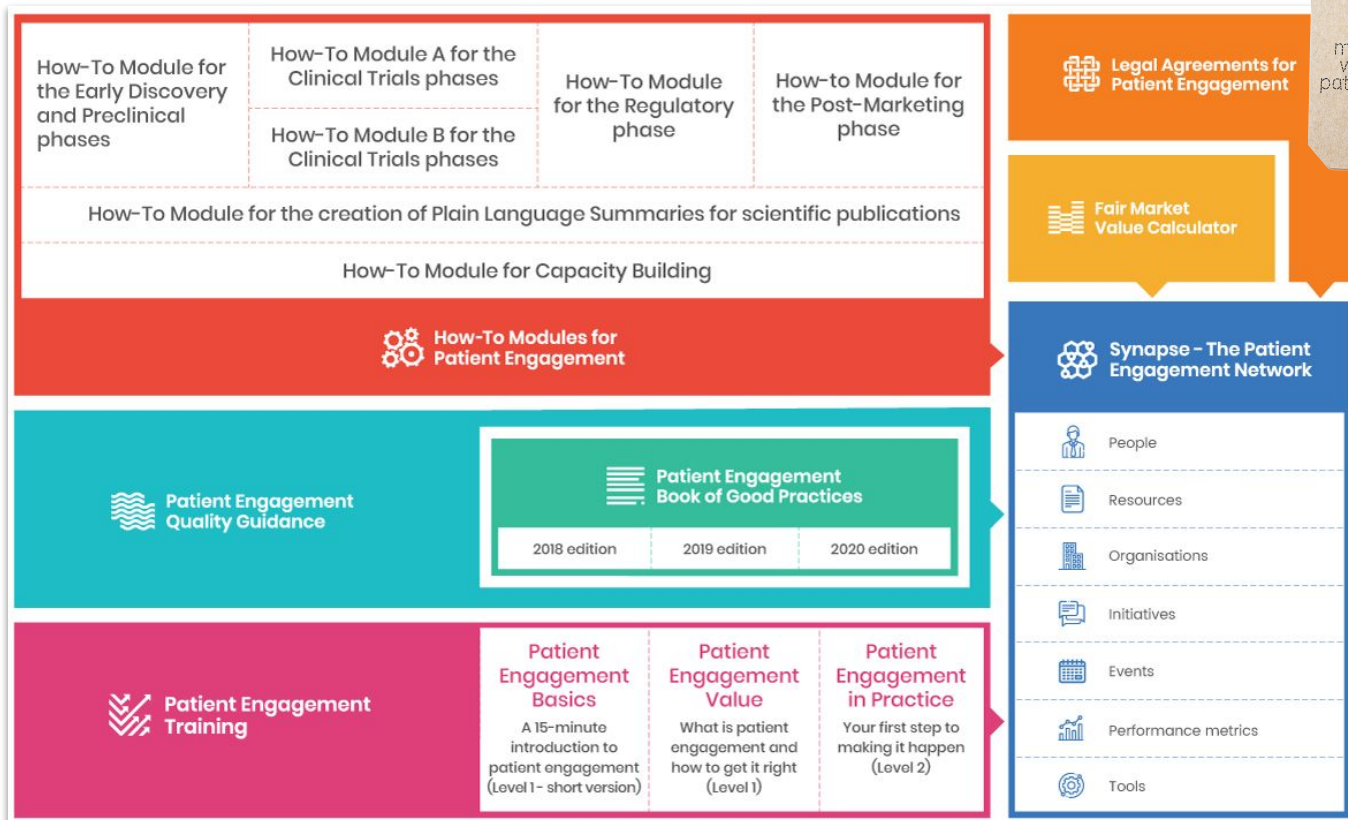
Executive Insight, Healthcare Consultants  
Parkinson's UK  
Commutateur Advocacy Communication  
Servier  
Ipsen Pharma  
Takeda

Healthivibe  
EATRIS  
Multi-Act  
Better Pharma Care Coalition  
eYPAGNet  
University of East Anglia  
National Kidney Foundation  
HTAi  
Multi-Act  
Takeda  
E18 Consulting  
Patient advocate/ expert/iCAN

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# Patient Engagement Management Suite

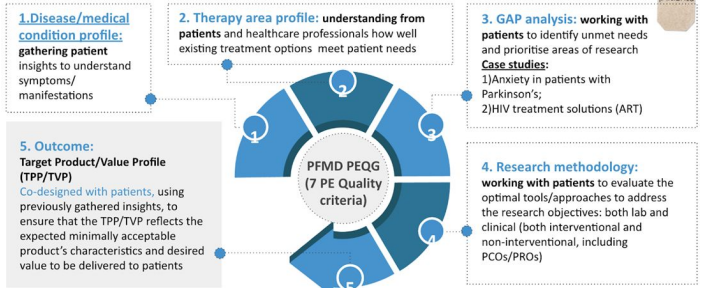


Accessible at [www.pemsuite.org](http://www.pemsuite.org)



# Evolution of the model with the feedback gathered

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# Deep dive into the sections of this how-to

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## HOW-TO GUIDE TO INVOLVING PATIENTS IN THE EARLY RESEARCH PHASES

Preparations for partnership and collaboration

Understanding condition and therapy area

Developing research methodology

Target Product and  
Target Value Profiles

**Presentation**  
**10-15'**

Use Q&A to ask questions and comment during the presentations

**Questions & Answers**  
**10'**

Q & A

**Polling**  
**5'**

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

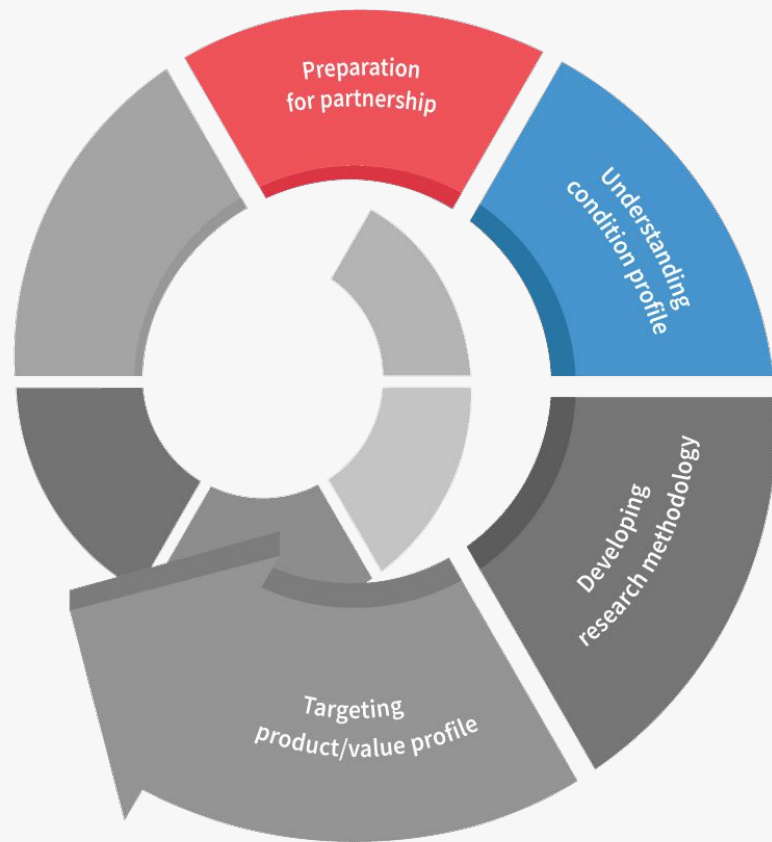


**Dr Oleksandr Gorbenko,**  
**MD, PhD**  
Global Patient Centricity  
Director IPSEN



# Preparation for partnership and understanding the condition profile

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# The importance - THE WHY

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**Discover the purpose** of your study

Patients are the best equipped to understand the condition

**Establish relationships** with patients / patient organisations

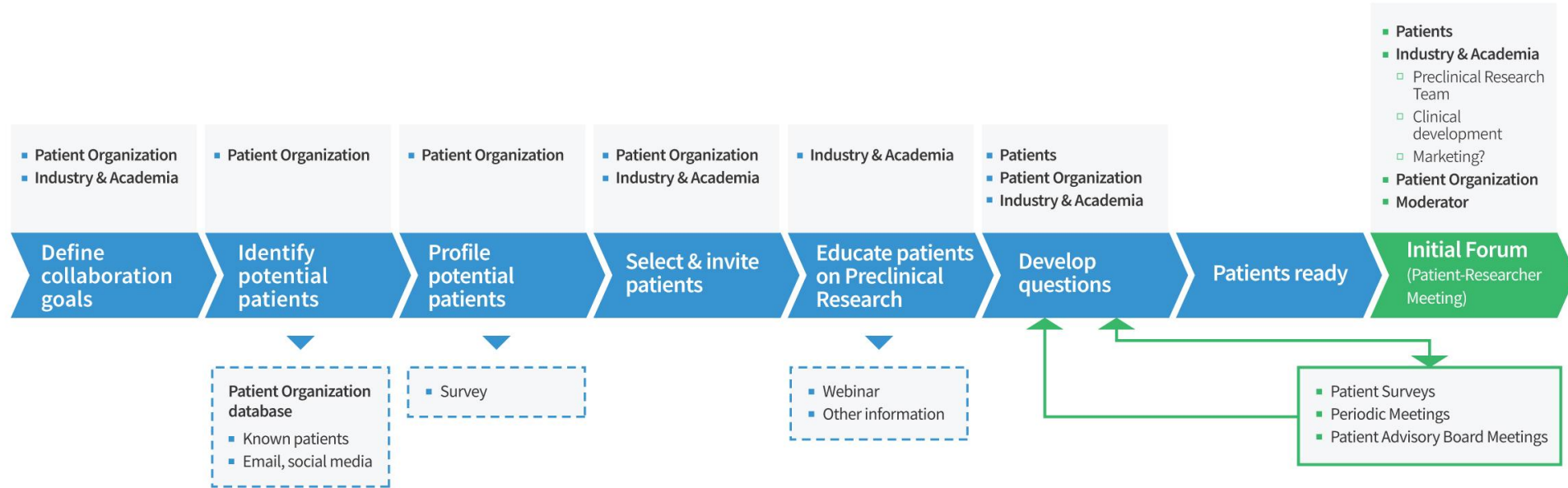
Engage to **discover patient viewpoints** of their condition and their ability to contribute

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

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## Condition profiling

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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**
- Discover the **'patient' experience** and perspective of what it is like to live with the condition

# Therapeutic area profiling

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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

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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

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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

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## 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?

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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

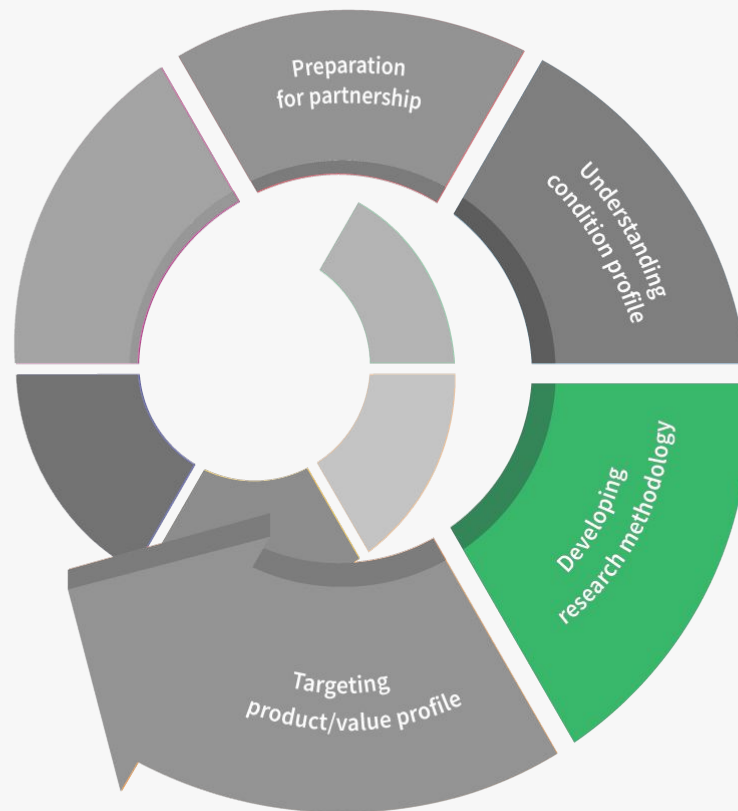


**Nick Hicks**  
Commutateur Advocacy  
Communications



# Creating a research methodology

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# 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

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## 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

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## 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

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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

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## 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

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## 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

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## 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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- Would this section be useful for you?
- 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

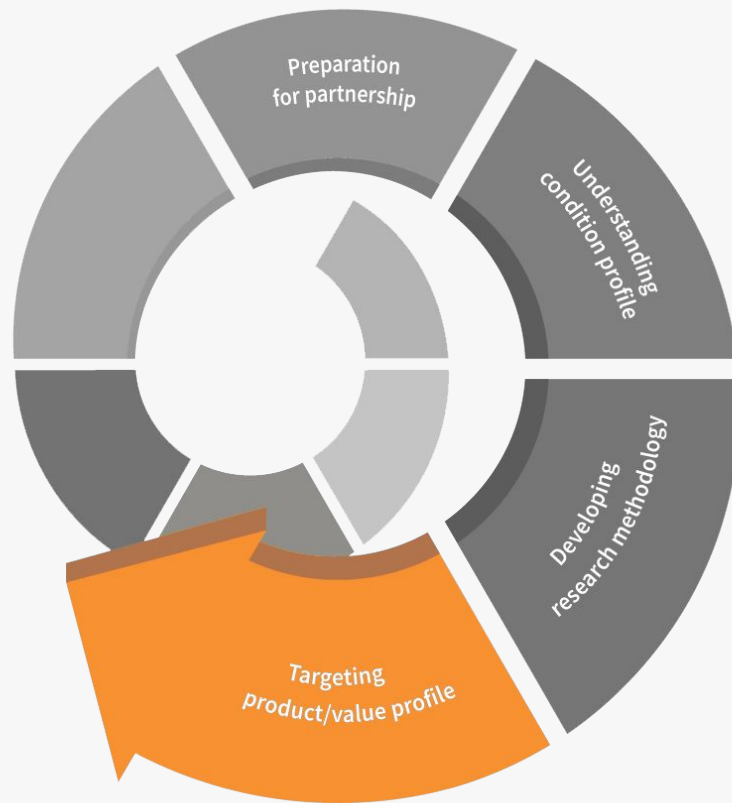


**Merlin Williams**  
Senior Consultant,  
Executive Insight,  
Healthcare Consultants



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

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

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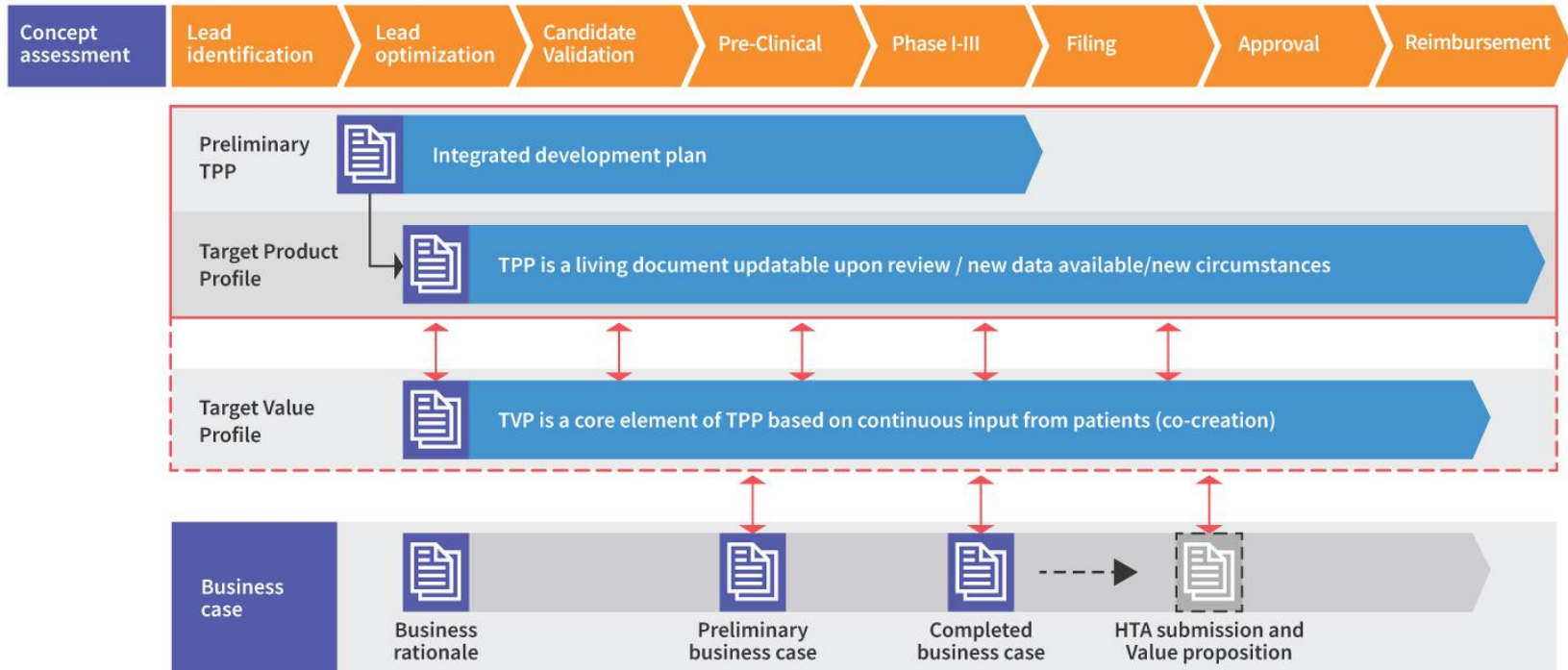
A **target value profile (TVP)** is an essential part of early drug development. It helps companies and researchers plan the development of a new medicine.

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

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# Key elements



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

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

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TPP element	What does it mean for developers (TPP)	What does it mean for patients (TVP)
<b>Tolerability profile</b>	<ul style="list-style-type: none"><li>● Non-inferior/superior tolerability profile in comparison of Standard of Care (reported as PRO);</li><li>● % of potential users adapting to tolerability issues within one round of use;</li><li>● % of discontinuation due to tolerability issues;</li><li>● No irreversible tolerability issue (issue that does not resolve after discontinuation of drug);</li></ul>	<ul style="list-style-type: none"><li>● <i>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)?</i></li><li>● <i>What kind of PRO/PCO measurements and tools should be used reflecting tolerability profile in the forthcoming studies?</i></li><li>● <i>% of study participants who have accepted/adapted to possible tolerability issues?</i></li><li>● <i>% of study participants who have discontinued due to tolerability issues?</i></li><li>● <i>Any expected irreversible tolerability issues?</i></li></ul>

## Example II: Dosage and administration under TPP and TVP

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TPP element	What does it mean for developers (TPP)	What does it mean for patients (TVP)
<b>Dosage and administration</b> (posology)	<ul style="list-style-type: none"><li>• Formulation/formulations;</li><li>• Types of administration/delivery;</li><li>• Injection site/sites;</li><li>• Injection volume;</li><li>• Dosing frequency;</li><li>• Number of pills per dose;</li><li>• Dosing timing;</li><li>• Dosing with relation to food;</li><li>• Dosing adjustments (see the factors above);</li><li>• Pill size;</li><li>• Coformularity - ability to be co-formulated into fixed dose combinations and/or single tablet/injection regimens;</li><li>• Other posology aspects for alternative formulations;</li></ul>	<ul style="list-style-type: none"><li>• <i>What are the most/least desirable formulations for this treatment?</i></li><li>• <i>What are the most/least desirable ways of delivery for this treatment?</i></li><li>• <i>Any changes in terms of formulations/ways of delivery vs existing SoC?</i></li><li>• <i>Desirable/acceptable injection sites?</i></li><li>• <i>Desirable/acceptable dosing frequency?</i></li><li>• <i>Desirable/acceptable number of pills per dose?</i></li><li>• <i>Desirable/acceptable dosing time?</i></li><li>• <i>Relation to food and drinks?</i></li><li>• <i>Relation to daily activities: physical, mental, sexual, working/daily routine, childbearing/breastfeeding?</i></li><li>• <i>Dependence from HCPs/clinics or caregivers in terms of administration/delivery;</i></li></ul>





# Q&A for Target Value / Product Profile 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?

## Next: finalising this work

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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](mailto:support@pfmd.org)

