Patient engagement in the early drug discovery phase: The role of Medical Affairs

Presented by the MAPS Patient Centricity Working Group and guest
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Presenters

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The following faculty and planning members do disclose financial relationships:

Dawn Lobban, Oleks Gorbenko, Dawn Richards

Educational objectives

Understand the need to engage with patients throughout the medicine development lifecycle, **starting early** during the discovery phase

Explore, **using case studies,** patient engagement in early development to understand disease burden, patient experience, unmet needs, and new treatment expectations to develop integrated asset / medicine development plans and target value profiles (TVP)

Highlight the **critical role of Medical Affairs** in initiating and developing patient partnerships at the discovery phase to ensure effective long-term collaborations

Gain **awareness of resources and best practices** to facilitate patient engagement in early drug development

eCAdemy

The role of Medical Affairs in driving patient centricity across the product lifecycle

Introducing the webinar series

Introducing the webinar series

The role of Medical Affairs in driving patient centricity across the product lifecycle

Discovery

Webinar 1: Early discovery

- Understanding patient experience and unmet need
- Developing Target Value Profiles

Clinical development

Webinar 2: Clinical development

- Optimizing clinical trial design
- PRO development / selection
- Patient preference studies

Pre-launch / launch

Webinar 3: Communication and education

- Communication of clinical data
- Patient education materials
- Publications PLS
- Disease awareness programs

Post launch

Webinar 4: Real-world evidence generation

- Patient support programs
- Real-world studies, PROs etc
- Ongoing unmet needs

Polling question 1

What is your level of experience of patient engagement within discovery and early development stages?

- I had no idea any patient engagement happens at this early stage
- Patients are involved at the early discovery phase in my company but Medical Affairs are not involved
- In my company Medical Affairs are involved in patient engagement at the early discovery phase

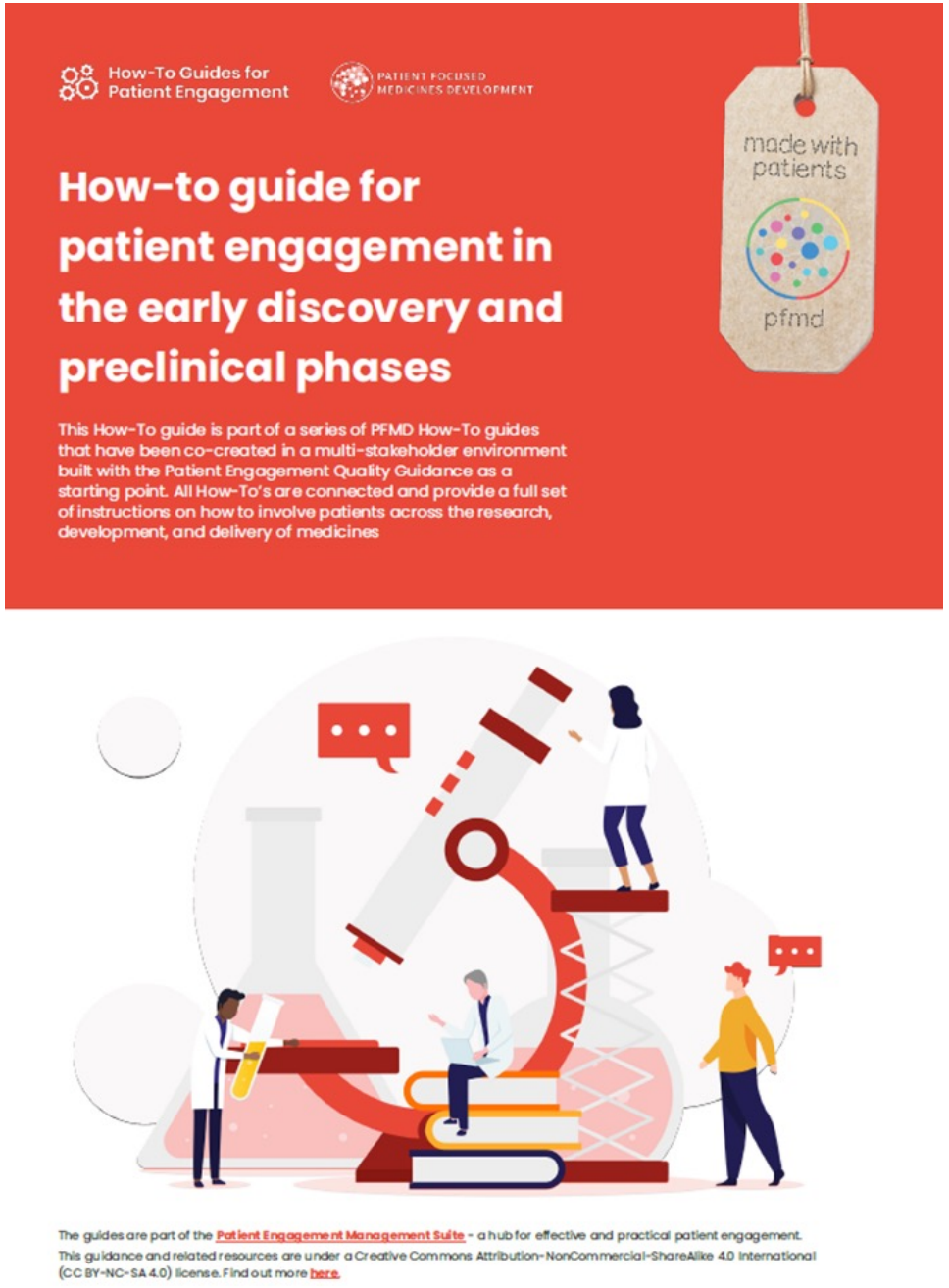
Polling question 2

Do you agree that Medical Affairs should be involved from the early discovery phase to coordinate engagement and facilitate advice-seeking and insights-gathering activities with patient experts?

- No – patient partners will add little value at this early stage
- Not really – patient input is important but this should be done by our colleagues in another function
- Yes – patient engagement at the early discovery phase is useful and Medical Affairs are well placed to do this

Patient engagement in the early drug discovery phase: The role of Medical Affairs

Oleks Gorbenko



PFMD "How to" guide

- First ever developed guidance
- Launched in June 2021
- Multi-stakeholder input, including patient experts
- Download the guide
  - https://pemsuite.org/How-to-Guides/Early-Discovery.pdf
- How-to guide for patient engagement in the early discovery and preclinical phase by PFMD is licensed under CC BY-NC-SA 4.0
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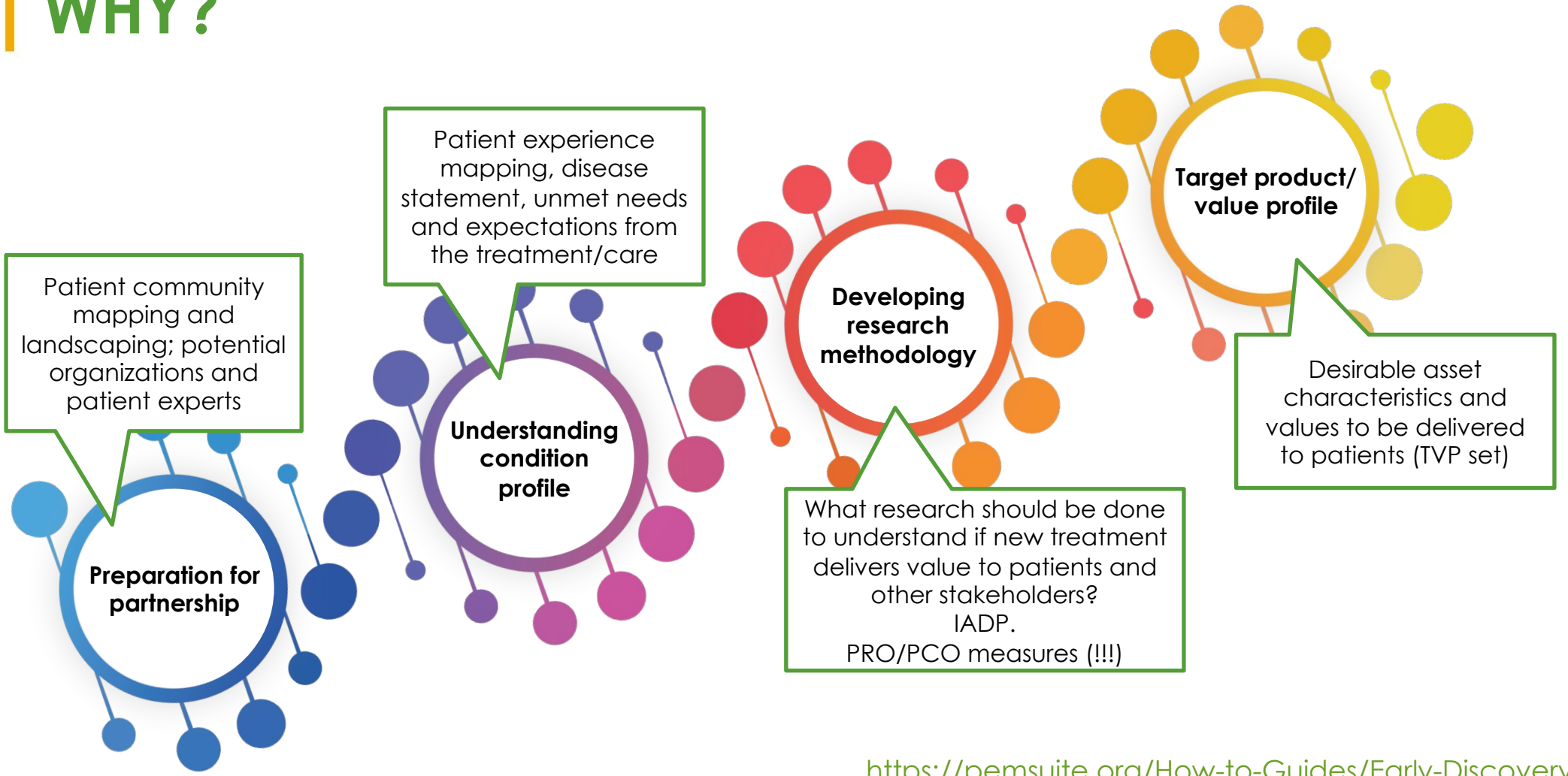
Start as early as possible!
WHY?



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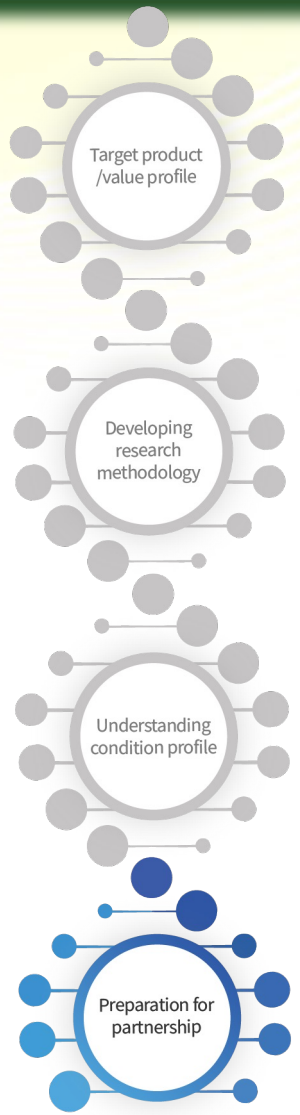
Stage 1 – Preparation for partnership

Dawn Lobban

Step 1: Preparation for partnership



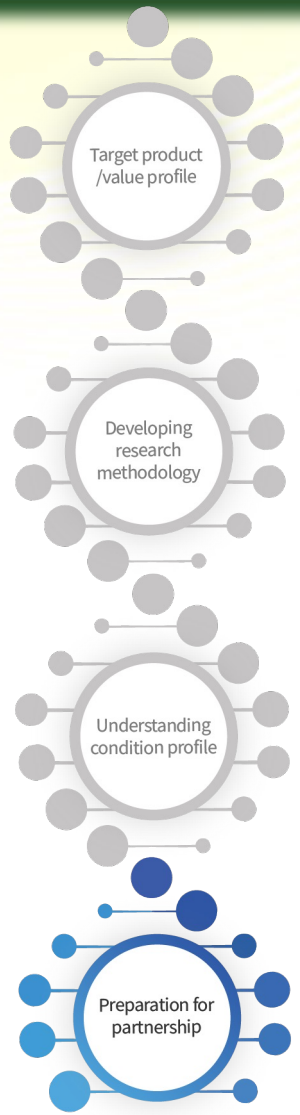
Goals of this step:

- **Identify opportunities** to work together
- **Prepare patients** and patient organizations
- **Prepare research team** for engagement
- **Co-develop a patient engagement plan**

https://pemsuite.org/How-to-Guides/Early-Discovery.pdf

Book 1, section 1 (page 13)

Step 1: Preparation for partnership



Remember:

- Show **reliability**
- Show **responsibility**
- **Trustworthiness** from all sides

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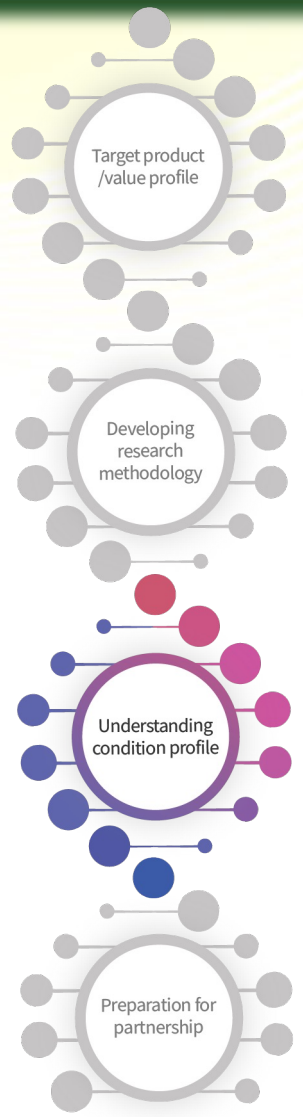
Book 1, section 1 (page 13)

Stage 2 – Understanding the condition profile

Dawn Richards

Step 2: Understanding the condition



The path to a patient-researcher exchange meeting

Stakeholders involved

1 Define collaboration goals	2 Identify potential patients	3 Select & invite patients	4 Help patients learn about preclinical research	5 Develop questions	6 Patients ready to be engaged	7 Patient-Researcher Exchange Meeting
▪ Patient organization ▪ Researchers	▪ Patient organization ▪ Researchers	▪ Patient organization ▪ Researchers	▪ Researchers	▪ Patients ▪ Patient organization ▪ Researchers		▪ Patients ▪ Researchers ▫ Other internal teams (where applicable)* ▪ Patient organization ▪ Moderator

Format of engagement

	Patient organization database ▪ Known patients ▪ Email, social media ▪ Survey to identify Patient Partners		▪ Webinar ▪ Other information	▪ Patient surveys ▪ Periodic meetings ▪ Patient advisory board meetings		

*Eg. Clinical development, Marketing department

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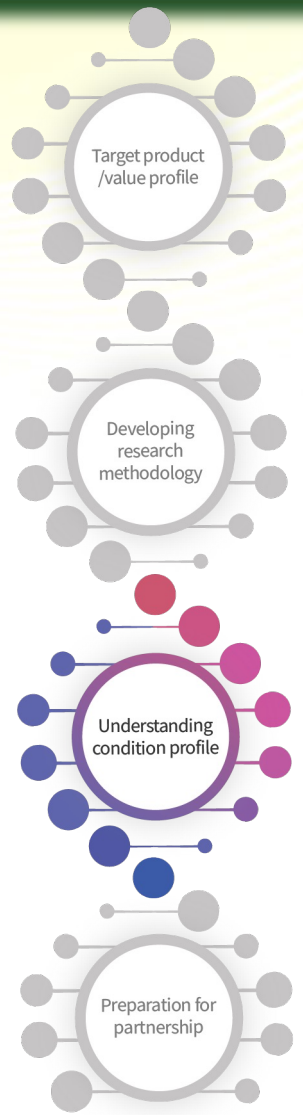
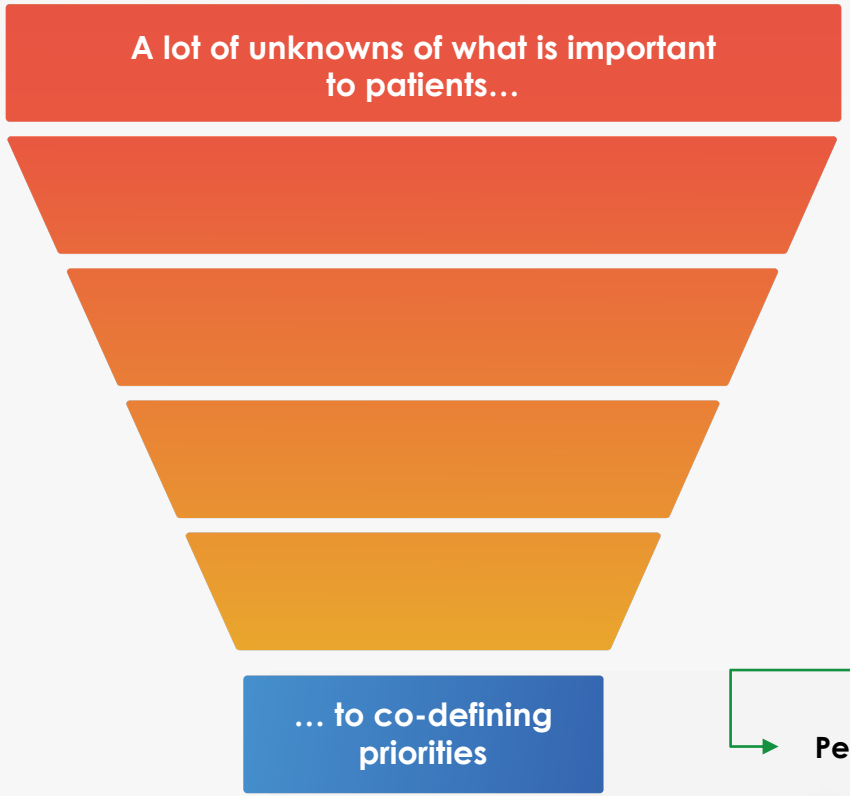
Book 1, section 2 (page 17)

Step 2: Understanding the condition



Evoking valuable patient insights to inform research



Example topics to understand:

- Living with the condition
- Challenges to treating the condition
- Gaps in current standard of care
- Patients' needs and preferences
- Risk-benefit profile and acceptable trade-offs
- Clinical trial preferences

See Book 2 page 49 – for more topics

Co-developing discussion questions

Performing gap analysis

https://pemsuite.org/How-to-Guides/Early-Discovery.pdf

Book 1, section 2 (page 17)

Stage 3 – Developing research methodology

Dawn Richards

Step 3: Goals for developing research approach

Some considerations/approaches to help you:

Evaluate **optimal tools and approaches** in lab-based studies

Evaluate studies to **address clinical questions and unmet needs**

Facilitate development of outcome measures for future clinical studies

https://pemsuite.org/How-to-Guides/Early-Discovery.pdf

Book 1, section 3 (page 23)

Step 3: Developing research approach

Some considerations/approaches to help you:

A steering group to oversee work

The patient-researcher exchange meeting to start the dialogue

- Format of discussions
- Probe the information you would like to learn about

Listen!

https://pemsuite.org/How-to-Guides/Early-Discovery.pdf

Book 1, section 3 (page 23)

Stage 4 – Target value profile (TVP)

Oleks Gorbenko

Step 4

Target value profile (TVP) versus target product profile (TPP)

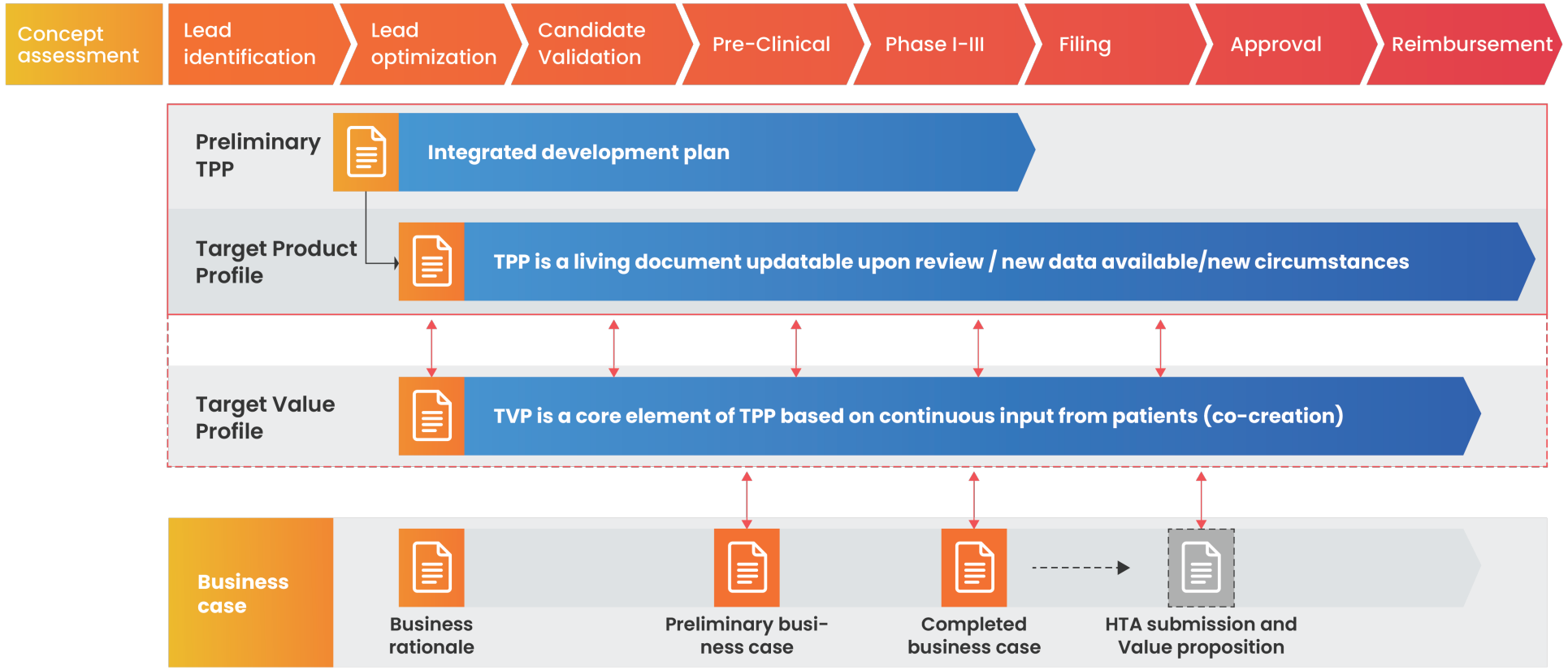
TPP	TVP
An updatable guidance for the industry / developers with targeted / desirable characteristics of a potential **product**	A consolidated set of **expected values** to be delivered to a patient by a chemical molecule, biological product, or medical device, used as treatments addressing areas of unmet needs

https://pemsuite.org/How-to-Guides/Early-Discovery.pdf

Book 1, section 4 (page 26)

Step 4

Target value profile (TVP) versus target product profile (TPP)



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Book 1, section 4 (page 26)

Basic TVP attributes

1. Efficacy and effectiveness
2. Resistance (for antimicrobial agents)
3. Safety profile
4. **Tolerability profile**
5. Clinical pharmacology
6. **Dosage and administration (posology)**
7. **Storage conditions**
8. Business rationale (business case – may/may not be a part of TVP)
9. **Value proposition / value summary**

https://pemsuite.org/How-to-Guides/Early-Discovery.pdf

Step 4

Product (TPP) and value (TVP) elements are similar, but characterized differently

	TPP	TVP
Tolerability profile	• Non-inferior / superior tolerability profile in comparison to SoC (reported as PRO) • % of potential users adapting to tolerability issues within one round of use	• What is the effect of a treatment's side effects on patient treatment continuation? To what extent can a patient tolerate them? • What would be a meaningful improvement compared to current therapies?
Dosage and administration (posology)	• Formulation / formulations • Types of administration / delivery • Injection site / sites • Injection volume	• What are the most / least desirable formulations for this treatment? • What are the most / least desirable ways of delivery for this treatment?

https://pemsuite.org/How-to-Guides/Early-Discovery.pdf

Book 1, section 4 (page 29)
Book 2, annex 4, (page 54)

Bringing the guidance to life – Real-world experience Medical Affairs

Four patient advisory board case studies

Oleks Gorbenko

Bringing the guidance to life
- Real-world experience Medical Affairs

Ipsen has agreed **to pilot** the guide with business processes and operations

Functions involved:

R&D (REED, Clinical Operations) and Global Medical Affairs / Global Patient Affairs

Started from:

Communication, cross-functional workshop and training

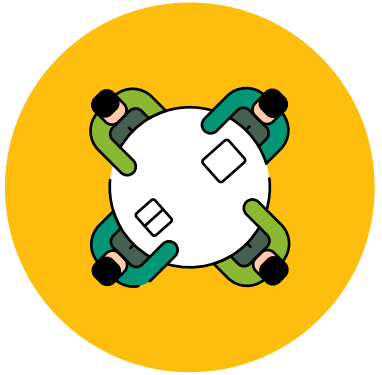
Personal experience:

Have facilitated four global patient advisory boards on new potential assets' TVPs (2018–2022):

- ART (anti-retroviral treatment, HIV / AIDS) x2
- Injectable treatment for chronic migraine
- Peroral treatment for levodopa-induced dyskinesia (LID) in Parkinson's disease

Case study:

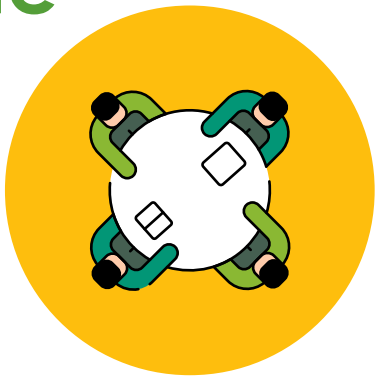
Peroral and injectable ART (anti-retroviral treatment, HIV/AIDS)



- Two global patient advisory boards on TVP for new peroral and injectable ART
- Patient experts, medical directors, and Research / R&D executives (decision-makers)
- Intensive discussion on nine TVP parameters
- Some parameters are more meaningful for PLHIV
  - tolerability, dosage, and administration
- Some parameters are less relevant
  - resistance, clinical pharmacology etc
- "Injectable (IV) ART may trigger relapse in injectable drug-users (IDU).."
- "6 ml is a huge injection volume for IM and could be very painful.."
- Such ARTs must provide undetectable viral load as quick as possible to ensure HIV untransmittable status in patients (sexual partners)

Case study:

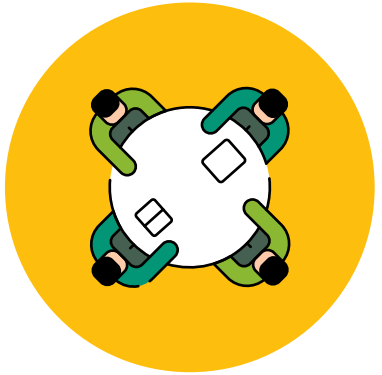
Injectable treatment for chronic migraine



- Global patient advisory board on TVP for new injectable treatment for chronic migraine
- Patient experience session before discussing TVP
- "We need more prevention, rather than episode-by-episode treatment.."
- Dear patient experts, what do you prefer (please vote and comment):
  - *To decrease severity of episodes?*
  - *To decrease frequency of episodes?*
  - *To decrease the need in acute painkillers?*
- Desirable injection sites?
- Tolerability: should be comparable with other treatment; injections must not trigger new episodes!

Case study:

Peroral treatment for levodopa-induced dyskinesia (LID) in Parkinson's disease



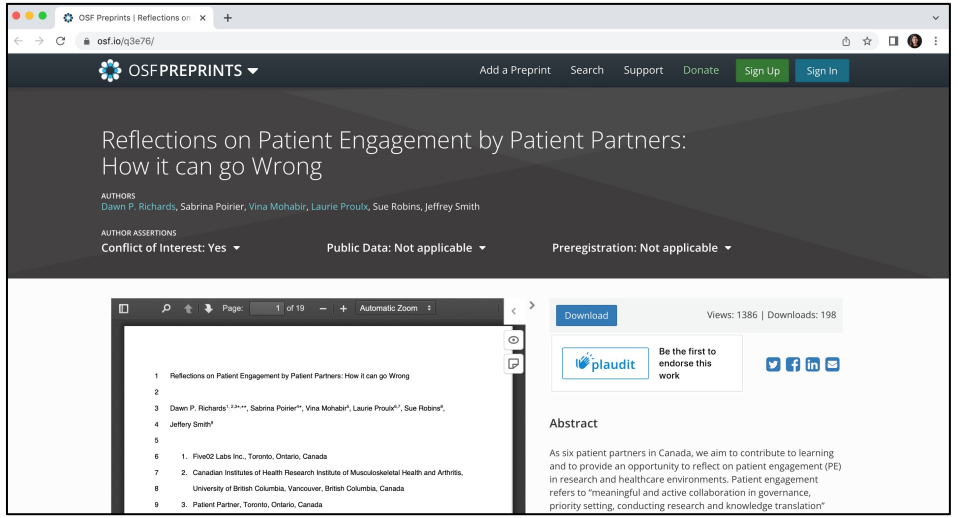
- Global patient advisory board on TVP for new peroral treatment for levodopa-induced dyskinesia (LID) in Parkinson's disease
- Patient experience session before discussing TVP
- To decrease hours per day (vs % of daily time) with dyskinesia
- It's appropriate to be slightly dyskinetic over the day (*how much time per day?*),
- HRQoL is crucial – treatment should maintain ability to work, travel, and socialize, especially for younger patients (no "windows")
- Physical appearance is important
- Safety: consider to change dramatically the levodopa treatment paradigm

Bringing the guidance to life – Real-world experience patient partners

Dawn Richards

“Good engagement” includes:

- Training for everyone on the team / who is part of the engagement
- Clear roles and responsibilities
- Transparent communication on compensation and expense reimbursement
- Lots of time to prepare
- A variety of activities and discussions (eg, don’t try to ‘make’ patient partners be researchers)
- The loop being closed in terms of where input went / how it influenced any outputs/outcomes
- Manage expectations
  - Risks and benefits
  - Transparency



A pre-print by six patient partner authors
https://osf.io/q3e76/

Some helpful resources

- PFMD 'How-to guide' for patient engagement in the early discovery and pre-clinical phases
  - https://pemsuite.org/How-to-Guides/Early-Discovery.pdf
- A how-to guide to patient engagement in research
  - https://cihr-irsc.gc.ca/e/27297.html#a2
- Matching researchers' needs and patients' contributions: practical tips for meaningful patient engagement from the field of rheumatology
  - https://ard.bmj.com/content/82/3/312
- Tokenism. Seeing It. Fixing It. Perspectives from IMHA patient partners
  - https://blogs.ubc.ca/imhablog/2021/10/13/tokenism-seeing-it-fixing-it-perspectives-from-patient-partners/
- Ethics guidance for developing partnerships with patients and researchers
  - https://cihr-irsc.gc.ca/e/51910.html

Panel discussion

Moderator: Dawn Lobban

Dawn to suggest – others to suggest more please

Questions for moderator to pose

For Oleks

- Medical affairs have lots of demands on their time, resources and budget – how would you convince them that early engagement with patients should be a priority?
- Tips for how medical affairs can work best with colleagues in R&D to get them on board with early patient engagement and the value of including MA in those initial interactions?
- How can medical affairs identify potential patient partners to engage with?
- How to you manage expectations of patients when engaging early about a product that might not make it?
- In your experience what is the patient partner response to being invited to be involved in early drug development?

Dawn to suggest – others to suggest more please

Questions for moderator to pose

For Dawn R

- How can Pharma find patient partners who may want to be involved in early phase drug development?
- In your experience what is the patient partner response to being invited to be involved in early drug development? How common is this?
- What do you think are the biggest barriers for patients to get involved in early drug development?
- How can we best support patient partners to develop the skills needed to participate?

Key takeaways for successful patient engagement in the early drug discovery phase

Preparations for partnerships	Understanding the condition profile	Developing research methodology	Target value profile
• Identifies the opportunities • Prepares all stakeholders • Co-develops an engagement plan	• Educates the researchers • Starts an ongoing dialogue • Identifies the gaps • Sets the research priorities	• Assesses optimal tools and approaches to meet research objectives • Agrees models, priorities and outcome measures	• Captures patients opinions, needs, expectations and preferences • Informs the target product profile

Thank you!