D5.1 Report on the first open Patient Engagement Forum

777450 - PARADIGM

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

WP5 – Dissemination and engagement

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Document History

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V1.3	22/05/2018	Draft
V2.0	28/05/2018	Final Version

Definitions

Partners of the PARADIGM Consortium are referred to herein according to the following codes:

1. **EPF**. EUROPEAN PATIENTS FORUM (Luxembourg) – **Project Coordinator**
2. **EURORDIS**. EUROPEAN ORGANISATION FOR RARE DISEASES ASSOCIATION (France)
3. **EATG**. EUROPEAN AIDS TREATMENT GROUP (Germany)
4. **AE**. ALZHEIMER EUROPE (Luxembourg)
5. **AIFA**. AGENZIA ITALIANA DEL FARMACO (Italy)
6. **HTAi**. HEALTH TECHNOLOGY ASSESSMENT INTERNATIONAL (Canada)
7. **IACS**. INSTITUTO ARAGONES DE CIENCIAS DE LA SALUD (Spain)
8. **FSJD**. FUNDACIO SANT JOAN DE DEU (Spain)
9. **VU-ATHENA**. STICHTING VU (The Netherlands)
10. **UOXF-CASMI**. THE CHANCELLOR, MASTERS AND SCHOLARS OF THE UNIVERSITY OF OXFORD (United Kingdom)
11. **EFGCP**. EUROPEAN FORUM FOR GOOD CLINICAL PRACTICE (Belgium)
12. **SYNERGIST**. THE SYNERGIST (Belgium)
13. **SYNAPSE**. SYNAPSE RESEARCH MANAGEMENT PARTNERS SL (Spain)
14. **EFPIA**. EUROPEAN FEDERATION OF PHARMACEUTICAL INDUSTRIES AND ASSOCIATIONS (Belgium) - **Project Leader**
15. **MSD Corp.** MERCK SHARP & DOHME CORP (United States)
16. **UCB**. UCB BIOPHARMA SPRL (Belgium)
17. **ABPI**. THE ASSOCIATION OF THE BRITISH PHARMACEUTICAL INDUSTRY (United Kingdom)
18. **AMGEN**. AMGEN LIMITED (United Kingdom)
19. **BAYER**. BAYER AKTIENGESELLSCHAFT (Germany)
20. **GSK**. GLAXOSMITHKLINE RESEARCH AND DEVELOPMENT (United Kingdom)
21. **GRT**. GRUENENTHAL GMBH (Germany)
22. **JANSSEN**. JANSSEN PHARMACEUTICA NV (Belgium)
23. **LILLY**. Eli Lilly and Company Limited (United Kingdom)
24. **LUNDBECK**. H. LUNDBECK AS (Denmark)
25. **MERCK**. MERCK KOMMANDITGESELLSCHAFT AUF AKTIEN (Germany)
26. **NOVO NORDISK**. NOVO NORDISK A/S (Denmark)
27. **PFIZER**. PFIZER LIMITED (United Kingdom)
28. **ROCHE**. F. HOFFMANN-LA ROCHE AG (Switzerland)
29. **SERVIER**. INSTITUT DE RECHERCHES INTERNATIONALES SERVIER (France)
30. **VFA**. VERBAND FORSCHENDER ARZNEIMITTELHERSTELLER EV (Germany)
31. **SARD**. SANOFI-AVENTIS RECHERCHE & DEVELOPPEMENT (France)
32. **NOVARTIS**. NOVARTIS PHARMA AG (Switzerland)
33. **COVANCE**. COVANCE LABORATORIES LTD (United Kingdom)
34. **ALEXION**. ALEXION SERVICES EUROPE (Belgium)

- **Consortium.** The PARADIGM Consortium, comprising the above-mentioned legal entities
- **Consortium Agreement**. Agreement concluded amongst PARADIGM participants for the implementation of the Grant Agreement. Such an agreement shall not affect the parties' obligations to the Community and/or to one another arising from the Grant Agreement.

1. Publishable Summary

The forum's opening comments were made by Nathalie Moll, EFPIA Director General, who explained that PARADIGM is a pivotal part of the EFPIA strategy and promoted active involvement and collaboration. This was followed by a series of partners. Nic*ola* Bedlington, EPF's Secretary General spoke of the need for synergies and avoiding duplication with other initiatives, such as Patient Focused Medicines Development and European Patients' Academy and stressed that speed and sustainability were imperative. These sentiments were echoed by Mathew May, Programme Coordinator EUPATI. He promoted collaboration and linkage across initiatives. The need for flexibility was touted by Magda Chlebus, Director Science Policy at EFPIA, in order to react to the changing landscape, she further expressed that this objective mirrors PARADIGM's remit. While Nicholas Brooke, CEO of PFMD recounted that patient engagement is an investment and there is an interest to expand past the innovators and early adopters, such as those at the forum, to collaborate to become a majority.

Various participants spoke of the need for conversion, to facilitate patient engagement becoming the norm, and some noted the requirement to demonstrate its added value. While different constituencies are represented, there is a shared focus on patient education and training, guidance on engagement, and ultimately sustainability. In the end, the ecosystem needs to be prepared, and fortification is derived from metrics. Defining metrics that are relevant to each of the stakeholders, who come to the process with often unique needs, is not an easy task. Co-creation is the pathway to sustainability, and finding synergies, while resisting competition is fundamental to success. PARADIGM provides a legitimate place where all stakeholders including regulators and industry can meet, learn from one and other and share best practice. EMA and HTA bodies can facilitate the advancement of patient engagement to a new level. ***Presenters****: Walter Atzori (Alexion), Pietro Mario Erba (AIFA), Juan García (EMA), Virginie Hivert (EURORDIS), Paul Robinson (MSD), Tjerk Jan Schuitmaker (VU Amsterdam) and Daniel De Schryver (JANSSEN).*

Four workshops were held to broaden stakeholder input: A. ***The Survey*** - Discussed the need to select the relevant questions, narrow down the questions for each stakeholder group and how to accomplish translation into other languages quickly; B. ***Building the PARADIGM Story*** – The storytelling team discussed the communications plan and the co-creation of the PARADIGM narrative, followed by breakout sessions that identified areas for operational excellence; C. ***Getting the Most of the Survey*** - An overview of some distinct considerations for people with cognitive impairment, rare diseases and children was presented. Input was gained on the best pathway to obtain appropriate industry responses for the survey; D. ***Assessing the Value of Patient Engagement*** -Through a short questionnaire and in discussion groups, participants identified the most feasible indicators to measure patient engagement at three decision points and possible types of case studies.

2. Methods

The PARADIGM consortium led by European Patients' Forum and EFPIA held its first forum on 10th of April 2018, which was co-organized by EUPATI (European Patients' Academy on Therapeutic Innovation) and PFMD (Patient Focused Medicines Development). A maximum of 2 representatives from each partner group attended along with key stakeholders. The representative groups included patient organisations, regulatory bodies, universities, non-for-profit organisations, SMEs, trade associations to pharmaceutical companies. The meeting consisted of presentations and workshops in which context for the project was provided and input on key aspects of the work was obtained.

3. Results

A key outcome from the forum was increased clarity on the objectives and the process that will be employed during the 30-months project. Participants discussed the survey with a broader group of stakeholders, explaining the methodology, and gaining input on the recruitment process.

The organizers obtained feedback on different ways to work with vulnerable populations, on ways to reach out to these communities both through existing networks and different structures and on ways to involve these vulnerable populations in the process.

The team also obtained input on key metrics at the three main points of the medicine lifecycle from the different stakeholder groups each provided varied perspectives. During research prioritization, possible indicators include whether the patient voice is reflected in the research agenda and the agenda is geared towards meeting unmet medical needs. There were more distinct criteria associated with clinical trials, including fewer protocol amendments, shorter timelines for the trial, faster and more diverse recruitments, more relevant end-points, increased patient satisfaction and earlier stop of unsuccessful research. Lastly, with respect to metrics for early dialogue with regulators and health technology assessment agencies, core measures were related to the early alignment of all stakeholders, as well as to the quantity of favourable reviews. Another point that was identified was some initial thinking on the types of case studies to be collected, this included anonymized retrospective examples showcasing outcomes with and without the inclusion of patient engagement.

The dissemination of information and input from various communities is a key aspect of PARADIGM. The need to build the PARADIGM story through co-creation and active engagement of partners was strongly voiced. Equally, the need to utilize the right voice and appropriate channels with respective stakeholders was articulated. The interest for a toolkit for PARADIGM partners with instructions and collaterals for external communications was viewed as being extremely valuable.

4. Discussion

The forum provided an opportunity for face to face interactions with a wide range of stakeholder groups. This created an avenue for alignment. The highly collaborative event modelled co-creation and stakeholder engagement. The inclusion of colleagues from PFMD, EUPATI and other patient engagement initiatives allowed for knowledge transfer, as well as the opportunity to build on existing successes. The sense of urgency was palpable and the work package representatives were invigorated by the interactions. The well-planned forum generated ideas to further the momentum of the project.

5. Conclusions

The 1st Open Forum on Patient Engagement is the first event of its kind, co-organized with other patient engagement initiatives; it merged a traditional kick-off meeting with a series of technical workshops. This format and the meeting outcomes were considered a success. The design will be repeated at the 2nd and 3rd Open Forums on Patient Engagement in order to enhance synergies and avoid duplication. Of course, there are learnings to be incorporated into future meetings. This includes the suggestion for the forum to be extended to one and a half days. Providing pre-read material to maximize the time in the meeting was also seen as valuable and appropriate for these later forums. Overall, the attendees and the consortium partners viewed the forum as a very fruitful event that provided an orientation to the entire project, context with respect to other patient engagement related initiatives, along with inspiration for continued success.

6. Annexes

- List of participants
- Workshop Outputs

1st Forum on Patient Engagement
List of Participants

- Sheuli Porkess ABPI
- Harriet Adams ABPI
- Ana Diaz AE
- Dianne Gove AE
- Pietro Mario Erba AIFA
- Giorgia Viceconte AIFA
- Walter Atzori ALEXION
- Mats Ericson AMGEN
- Pooja Merchant BAYER
- Matthias Gottwald BAYER
- Wolf See BAYER
- Jackie Dudley COVANCE
- Maria Dutarte EATG
- Giorgio Barbareschi EATG
- Emilie Fillod EFGCP
- Ingrid Klingmann EFGCP
- Magda Chlebus EFPIA
- Zsofia Bakonyi EFPIA
- Kirsty Reid EFPIA
- Nathalie Moll EFPIA
- Juan Garcia Burgos EMA
- Mathieu Boudes EPF
- Matthew May EPF
- Valentina Strammiello EPF
- Nicola Bedlington EPF
- Letizia Gambini EPF
- Anna Trzcinska EPF
- Zilvinas Gavenas EPF
- Sara Gayarre EPF
- Dominique Hamerlijnck EUPATI Alumni
- Viktoria Fonsou EUPATI Alumni
- Joan Jordan EUPATI Alumni
- Virginie Hivert EURORDIS
- Elisa Ferrer EURORDIS
- Begonya Nafria Escalera FSJD
- Joana Claverol Torres FSJD
- Lars Joens GRT
- Deborah Russell GRT
- Kay Warner GSK
- Paulin Kitchiner GSK
- Sandra Garcia IACS
- Maria Jose Vicente-edo IACS
- Iwona Jablonska IMI
- Daniel De Schryver JANSSEN
- Robert Kroes LILLY
- Gianluca d'Anzeo LILLY
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- Paul Robinson MSD
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- Kirsty Reid EFPIA
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- Mathieu Boudes EPF
- Matthew May EPF
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- Nicola Bedlington EPF
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- Jan Geissler PFMD
- Helena Harnik PFMD
- Søren Eik Skovlund PFMD
- Danielle Barron PFMD
- Tony Hoos PFMD
- Roxana Radu PFMD
- Diane Driver PILG representing Transcelerate
- Christina Åkerman PILG representing ICHOM
- Birka Lehmann PILG as external expert
- Inka Heikkinen PILG representing DIA
- Daniel O'Connor PILG representing MHRA
- Julia Chamova PILG representing ISPOR
- Jürgen Kübler PILG representing the IMI project PREFER
- Isabelle Huys PILG representing the IMI project PREFER
- Violeta Stoyanova PILG as external expert
- Rebecca Vermeulen ROCHE
- Vinciane Pirard SANOFI
- Mathieu Jouannin SANOFI
- Juliette Maillet SERVIER
- Anne-Claire Julienne SERVIER
- Eva Molero SYNAPSE
- Luca Bertoglio SYNAPSE
- Nicholas Brooke SYNERGIST
- Chi Pakarinen SYNERGIST
- Daphnee Pushparajah UCB
- Laurence Leonardy UCB
- Nicholas Fahy University of Oxford
- Suzanne Ii University of Oxford
- Barbara Haake VFA
- Tjerk Schuitmaker VU-ATHENA
- Nicole Goedhart VU-ATHENA

WORKSHOPS

Workshop A. *The survey in PARADIGM: prioritization of the survey topics and questions*

Publishable Summary

The workshop provided an opportunity to gain broader input on the survey including the methodology, recruitment process and determining prioritisation of survey questions. Excellent input was obtained, but there was no consensus on the prioritisation of the questions.

Key Objectives

Key Objectives
1. To explain the methodology behind the survey
2. To look at the recruitment process for the survey
3. To carry out a prioritization exercise for the survey

Summary of Key Discussion Points per Objective

Summary of Key Discussion Points per Objective	
Objective Number	**Key Points**
1	• Targeted recruitment (30-40 per target group) (snowball sampling) • Open recruitment – openness and volume – survey publicly available (everybody can participate). To address comments from the reviewers to reach out to stakeholders outside the consortium • Data will be broken down by these two groups
2	• Piloting Identify within PARADIGM 1 representative from each stakeholder group • Validation exercise to test language, readability To be done before going live in May • Recruitment commitment We need you! – all partners and PILG

3	Prioritisation exercise
	• Split into groups of 7-9 with balanced representation for stakeholders
	• You will receive the survey questions for one of these groups with these number of questions:
	  ○ Patients (21)
	  ○ Industry (18)
	  ○ Regulators (25)
	• Your mission is to select only 15 questions, out of which 2 can be free text in the next 35 minutes
	• Choose a rapporteur and a note taker
	• Please present which questions you took out and why

Core

Conclusions

Core Conclusions

Overall outcome:
• Key challenge is not just the content of the survey, but also the process of recruitment over a pretty short timetable in order to get sufficient levels of participation to give a meaningful benchmark of existing needs.
• It is useful to have translation of the survey, to help ensure broad coverage.
• It is also useful to reduce the total number of questions to closer to 15, in order to have a reasonable respondent burden.
• There were lots of very useful feedback, the need to streamline from five working groups, was identified, but no consensus at this stage.
• There was agreement on the need to move forward, and so we will:
  ○ Welcome written feedback from and immediately after today's session;
  ○ Pilot a revised version of the survey;
  ○ Circulate a final revised version for comments; and,
  ○ Then proceed to carry out the survey, aiming to launch in May.

Attachments Slides

PARADIGM First forum_Wkshop A_The

Workshop B. *Building the PARADIGM story*

Publishable Summary

In this workshop we provided the overall context of the external communication of PARADIGM and presented an overview of the Communications Plan: why we need one and how we have translated the project objectives into communications objectives; the key messages we have for each of the main target groups identified. We also presented the concept of the storytelling team, serving the ongoing co-creation of the narrative around the PARADIGM story.

To facilitate input from all the participants we had breakout sessions on brainstorming and discussion/feedback on common voice, storyline, channels, main topics and speaking opportunities.

We split in three groups during the breakout sessions. The groups offered many ideas. Key discussion points included the need to build the PARADIGM story through co-creation and active engagement of partners, to engage on appropriate channels with all stakeholders, to make the most of external speaking opportunities, to create a toolkit for PARADIGM partners with instructions and collateral for external communications.

Key Objectives

Key Objectives
1. Provide overall context of external communication of PARADIGM
2. Present an overview of Communications plan and the storytelling team and process
3. Content creation for PARADIGM (brainstorming, discussions and feedback on common voice, storyline, etc.)
4. Gain detailed feedback on Communication plan after the Forum

Summary of Key Discussion Points per Objective

Summary of Key Discussion Points per Objective	
Objective Number	**Key Points**
All mainly 3 & 4	Communications need to be co-created with and by partners
	How to reach outside of the known PE enthusiasts and unaffiliated patients
	Need to make sense of different initiatives in the patient engagement landscape and where PARADIGM fits
	Learn from partners and other projects, e.g. learning about live info flow or early and broad engagement of communities
	Wise use of events, determine how to work with other initiatives and partners at relevant speaking engagements: Prioritise events where we want to be
	Ongoing creation of communication materials, keeping in mind the right messages to the right audience, the need for translation and the level of information
	Connect to partners' communication resources to deliver the PARADIGM messages
	Participate in public consultations, e.g. in FDA request for responses for guidance
	Engage with external groups to build interest in patient engagement (e.g. University of Lyon's young HCPs)
	Managing negative public perceptions of patient - pharma collaborations→

	PARADIGM to provide some instructions on how partners should talk about PARADIGM and collaborate
	Awareness campaign to inform the public that patients have a voice and are heard → empowering patients to take action in their healthcare
	Clarify what is the role and output of PARADIGM ethical board
	Toolkit, instructions and collateral for partners (+PILG and other boards) to use in external communication • engagement/ outreach strategy and how partners can support • guide how to motivate/ best reach partner organisations' internal audiences
	Focus on "hard facts" when communicating about PARADIGM outputs (also as an instruction to the storytelling as well), using examples from individual's partners, to make communication more robust and credible
	Reuse and repurpose existing communication material and testimonials to amplify the PE message → creating healthy competition via FOMO
	Creating opportunities or facilitate engaging conversations for PARADIGM partners and others
	Create ownership of PARADIGM through the development of every individual to become an ambassador for PARADIGM
	Other topics: • Market access for patients after trials • Pricing and reimbursement

Core Conclusions

Core Conclusions
• Truly utilise the PARADIGM members and network in order to accomplish goals
• Create fresh content and toolkits during the course of the project
• Use varied channels for outreach that can reach different communities
• Wise use of events and purposeful prioritization

Attachments

List of Attachments

- Agenda
- PowerPoint presentations

Attachment 1) Agenda

- Setting the stage
- Communication Plan
    - A compass for our work
    - Why a communication plan
    - Translating project objectives to communications objectives
    - Key target groups, key messages
- From Communications to Engagement
    - The storytelling team
- Breakout session: What do you want to tell, to whom, when.
- Wrap-up

Attachment 2) Slides

WP5 Workshop - Forum_ALL.pptx (1).p

Workshop C. *Getting the most of the survey - which stakeholders and what methodologies*

Publishable Summary

The session addressed the different ways to work with vulnerable populations in Patient Engagement and how to reach out to them through the existing networks and new structures. Vulnerable populations will be involved in WP1 in different ways, including participation of patient representatives in the survey and of individual patients in the Delphi process and in a qualitative face-to-face consultation later in the year. The main questions and techniques that will be used for these face-to-face consultations will be developed in collaboration with the relevant members of the consortium.

Key Objectives

Key Objectives
1. Explain the importance of obtaining the views of vulnerable patient groups (young people, people with dementia and people living with rare diseases)
2. Present the respective methodologies to get the views and how to reach vulnerable populations to be involved in the different activities of the project
3. Map and identify the relevant persons/functions within pharmaceutical companies, from whom it would be important to have an answer to PARADIGM WP1 survey

Summary of Key Discussion Points per Objective

Summary of Key Discussion Points per Objective	
Objective Number	**Key Points**
1	• People with dementia have been traditionally excluded from patient engagement activities. The relevance of involving people with dementia has been recognised, but to ensure meaningful involvement of this population their specific needs have to be addressed. • Patient engagement (PE) of people living with rare diseases (PLWRD) in medicines R&D is important due to the low number of therapeutic options available but may be hampered by the low number of patients for each rare disease and the scattered expertise and resources. • PE of young people is important to address the following issues: prominent use of off-label treatments that are authorised treatments for adults, dosages/formulations not addressed to children, limited experience in paediatric medicines research (only 50 % of treatments addressed to children and young people, 90 % of treatments addressed to neonates). Involvement of children and young people along the lifecycle of medicines is feasible.
2	• The EWGPWD (European Working Group of People with Dementia) will participate in a workshop in June, the techniques to be used have been already tested and will depend on the issues to be addressed experience (brainstorming, open discussion based on questions and

	prompts, prioritisation using oval mapping, dots or archery, and other techniques for sensitive, difficult or abstract topics, like vignettes or extracts from media). • Children and Young Persons' Advisory Group (YPAG) to involve young people in health research. The group is formed by patients and non-patients, a facilitator and a clinical researcher. • The views and preferences of PLWRD have been since years gathered to inform EURORDIS advocacy work through surveys and focus groups and more recently through the Rare Barometer Programme. For the objectives of WP1, the EURORDIS membership will be targeted, together with more specific groups (Therapeutic Action Group, EURORDIS Summer and Winter School alumni, European Patient Advocacy Groups within the European Reference Networks).
3	Pharmaceutical companies are often large organisations with a complex structure. Teams and departments might work in silos and communication across functions is not always seamless. For the purpose of the survey it will be critical to have the feedback and answers from all the relevant expertise within companies, and we will need to target the right departments to ensure they answer the survey. It will be key to go beyond the "usual suspects", like members of the Patient Engagement Team, and have feedback also from more operational departments. In order to understand where the workshop participants saw the most relevant contacts sitting within companies, each attendee had to identify three industry functions (or alternatively departments or teams) taking into account the three priority areas that the PARADIGM project is focusing on. Results of this exercise showed the R&D department is the most relevant to target, followed by Patient Engagement team and others such as Public and Government Affairs.

Core Conclusions
The session addressed the different ways to work with vulnerable populations in Patient Engagement and how to reach out to them through the existing networks and different structures. Vulnerable populations will be involved in WP1 in different ways, including participation of patient representatives in the survey and of individual patients in the Delphi process and in a qualitative face-to-face consultation later in the year. The main questions and techniques that will be used for these face-to-face consultations will be developed in collaboration with the relevant members of the consortium.
Pharma companies are very complex and sometimes are divided in silos. The second part of the workshop was dedicated to mapping out and understanding better the departments and people within pharma companies who should complete the survey. Within pharma companies R&D seemed to be the department where most people would contact for questions related to PE, taking into account the three steps identified by PARADIGM. Within R&D, Clinical trials department and pre-clinical departments were identified as to be specifically targeted by participants. Other departments were identified, including Patient Engagement teams and Public Affairs but, the conclusion that it is difficult to have a clear pattern, and it was agreed that the approach should be as broad as possible to make sure that there is as many relevant answer as possible.
After the consolidation of the presentation and the mapping exercise made during the workshop, all partners of PARADIGM will need to commit to the spreading of the survey and make sure that the identified relevant networks / teams / expertise answer the survey to ensure useful feedback from the survey.

Attachment: Slides


PARADIGM First forum_Wkshop getting


Getting the most out of the survey - Industr


FSJD 1st forum.pdf


AE 1st forum.pdf

Workshop D. *Assessing the value of patient engagement*

Publishable Summary

In the workshop assessing the value of patient engagement, co-organized by WP2 & WP3 we focussed, first, on overcoming possible issues regarding the alignment of WP2&3. This was a fruitful discussion and no major issues are currently at hand.

Following that, all 31 participants filled out a short questionnaire in which they defined from the perspective of their background or organisation what the value of patient engagement is in the three decision-making points and how to measure that; what are good indicators? Furthermore, they defined what could be called "Return on Investment" (efforts, time, for each stakeholder) in each decision point. Based on this input we discussed and prioritized the most important and feasible indicators for measuring patient engagement practices. For Research priority setting possible indicators include whether the patient voice is reflected in the research agenda and the agenda is geared towards meeting unmet needs; for (design of) clinical trials these include fewer protocol amendments, shorter timeline of CT, faster and more diverse recruitments, more relevant end-points, increased patient satisfaction and earlier stop of unsuccessful research; and finally for early dialogue (with regulators and HTA) the most important are early alignment of all stakeholders and turning a possible 'no' into a 'yes' when there are unmet needs.

Overall, we gathered useful leads on concrete indicators.

We concluded the workshop by a group discussion on possible profiles of cases. We are looking for 1) retrospective cases (anonymized) which ran with patient engagement, 2) retrospective cases (anonymized) which ran without patient engagement (We can include the opinion of patients into those cases input. What would have been different?) and 3), prospective cases. We gathered good suggestions but need to better define what the profiles are.

Key Objectives

Key Objectives
1. Build consensus on key issues related to alignment WP 2&3
2. Collecting perspectives of the involved stakeholders on feasible and valuable indicators of patient engagement
3. Building a profile of cases

Summary of Key Discussion Points per Objective

Summary of Key Discussion Points per Objective	
Objective Number	**Key Points**
1	Explanation that WP2 focus on 'Good Engagement Practice'. WP2 will learn from previous projects and will heavily rely on PFMD. WP2 will do a gap analysis. What is missing? This will be integrated with the work of WP1. Building on this, WP3 focus on the measurement: how can we measure value of patient engagement? The main difference is that WP2 focusses on valuable process we can use as best practices and WP3 on good outcome indicators. Distinction between both is clear for the audience. No questions. In the proposal the concepts value, impact and ROI are used interchangeably. The question is, what is value? Everyone has a different perspective on the

	definition of value. The audience indicates that it is important to have a definition of value, otherwise you do research into non-corresponding areas. The facilitators indicate that this is one of the key point of the breakout session (=objective 2). Another issue that is raised is whether we want to use ROI (Return of Investment) or ROE (return on Engagement). Industry/companies mention that is recommended to use ROI. Moreover, patients invest also "time" in patient engagement. So, for patients the term is also workable. However, ROE is for PARADIGM the key term and therefore WP3 will work with that term to evaluate. ROI will be taken into account because of the value for the industry. We should constantly be clear about the terms we use. Last issue raised was how the survey of WP1 is now will link to the work of WP2 and WP3. The output of this survey will be used to finalize the questions for the survey of WP1.
2	In order to have sustainable patient engagement practices, it needs to be valuable for all stakeholders involved. This session's aim is to define value from the perspectives of the relevant stakeholders and elicit corresponding indicators. All attendees filled out a short questionnaire about the value of patient engagement. We focus on outcome indicators, not process indicators. Process is about meaningfulness of patient engagement, outcome, about measurable impact. Based on the answers in four groups the indicators are prioritized. **Group 1**: Indicators for value of patient engagement in the priority setting phase: • Project selection is driven by unmet needs. Project selection starts from the beginning with engagement of the patient. We should measure what the impact is of early engagement of the patient. • That engagement really happens and that it happens early. • Alignment on the priorities. Across stakeholder group, not only between the industry and patient, but that the unmet needs are recognized by the wider community. • Effect on the go/no-go decision. Patient voice is actually reflected in the go/no-go decision. • Use the Net Promotor Score (NPS) or Net touch point score. You ask each stakeholder what they think was their impact of their involvement. You can measure alignment of all stakeholders in a very simple way. **Group 1**: Indicators for value of patient engagement in the clinical trial phase (not plenary discussed) • Lower recruitment time, more diversity in recruitment, fewer protocol amendments • Retention rate, Better trial set up = continued participation • DVPT time, go/no-go faster • ROI for patient commitment • Site burden lower, less informed consent changes, design of clinical (Quality of Life, real informed consent, language, enough time to read + decide respect patient)

Group 1: Indicators for value of patient engagement in the HTA phase (not plenary discussed):

- Patient value parameter

Group 2: Indicators for value of patient engagement in the priority setting phase:

- Whether the mapping of patient journey is actually done.
- Demonstrating inclusion of patient needs and preferences in decision making. How it is demonstrated is quite hard to measure.
- Satisfaction of the patient for the time and efforts spent in interacting with the other stakeholders.

Group 3: Indicators for value of patient engagement in the design of clinical trials:

- Feasibility of the trial. This can be measured for example as speed of enrolment completion.
- Choices of the endpoint and the relevance of the endpoints. For example, for patient in terms of burners and feasibility. This can be measured as number of PRO (patient reported outcomes).
- Patients preference. Is that reflected in clinical practice? This is more in terms of uptake or post-marketing organisation, post launch. So, the uptake of the outcome. Patient engagement can for example change the acceptability of the medicine. You can compare projects with and without patient engagement. Is the uptake/adherence better in projects with patient engagement? Recommended article from the audience: Levitan, B., Getz, K., Eisenstein, E. L., Goldberg, M., Harker, M., Hesterlee, S., ... & DiMasi, J. (2017). Assessing the financial value of patient engagement: a quantitative approach from CTTI's Patient Groups and Clinical Trials project. Therapeutic Innovation & Regulatory Science, 2168479017716715.

Group 4: Indicators for value of patient engagement in the priority setting phase:

- Uptake of a product in the market.
- Efficiency of how you make the go/no-go decision. Killing something quickly is an efficient way of using your resources. This can be maybe measured to take a look to cases which are stopped due to the involvement of patients.
- Understanding patients' needs and reflection of the need in product profile.

Group 4: Indicators for value of patient engagement in the early dialogue phase (regulators and HTA) (not discussed):

- Helps HTA/Health care system to value outcomes more fully
- Positive decisions more likely based on QoL/PRO's
- Extension of decision to patient groups not studied b/o patient engagement.

Patient engagement in the early dialogue phase can have an impact on decisions whether to reimburse a project. If there is value for the patient a possible *no* can be changed in a *yes*. But if the costs are high and there is a lot of uncertainty and the patient don't see the value than it is definitely a *no*. This

	is a good lead to further investigate in term of measurability? All groups were struggling with the fact that they were more focused on processes instead of outcomes. It is hard to think of immediately measurable outcomes. But if we want to convince the outside world we need to come with clear outcome indicators: this is good for health care in general or this is good for "my tax money". It is not possible to measure those outcomes in the time frame of PARADIGM. However, WP3 approaches this by working with a realist evaluation framework. This framework indicated that mechanism (or processes) lead in a specific context to specific outcomes. Outcomes are something we desire but we will not know in the near future. WP3 will work on this the coming 30 months.
3	We need to understand what is going on with patient engagement in the field at the moment. Therefore, we are looking for ongoing research in which patient are or will be engaged. With those prospective cases we like to find who are engaging in clinical trials? In which phase are the patients included? Besides, we are looking for retrospective cases which ran with patient engagement. We are interested in how impact of patient engagement is measured. What matrix is used? Is the patient voice reflected in the decision points? If not: why not? Last, we are looking retrospective cases which ran without patient engagement. We know what happened in this case (engagement, drop-out, impact, etc.). We would like to model what would happen if you include patients. So, we can compare the baseline with the model. The audience mention some examples of cases WP2 can probably use. From the discussion, it became clear that we need to define which cases we want and share that with the patient organisations and the industry. The description of the case need to be short and make clear all the conditions of the cases.

Core Conclusions
WP2 & 3 seems good aligned. No major issues.
The workshop gave valuable input for WP3. Based on the discussion and answers we have leads, e.g. NPS, efficiency go/no-go decision, reflection of patient preference.
The workshop gave insight that there is a need for a short and detailed description of the needed cases need. This description need to be distributed in the industry/ patient organizations/ hospitals/ etc. No major concerns that there will be not enough cases available.

Attachments

List of Attachments

- Agenda
- PowerPoint presentations
- Action Steps per Objective

Attachment 1) Agenda

1. Opening
2. Alignment WP2 & WP3
3. Valuable and feasible indicators for patient engagement
4. Case selection

Attachment 2) PowerPoint presentations

20180410 workshop PARADIGM WP2&3.ppt

Attachment 3) Action Steps

Task/Milestone	Target Date	Who	Details
Report back to WP5 for dissemination	13th of April	Tjerk Jan, Nicole	Results of analyses are reported back
Analyses of workshop	30th of April	Tjerk Jan, Nicole,	Eliciting most relevant and feasible indicators
Adaption of survey questions based on the workshop	16th of April	Tjerk Jan, Nicole	Based on the indicators discussed in the workshop we rephrase the questions.
Create document profile of cases to send to the industry	30th of April	WP 3. Lead: Tjerk Jan, Nicole. Together with Paul and Lars	This document contains starter information on possible cases.