PARADIGM

Patients Active in Research And Dialogues for an Improved Generation of Medicines
Webinar to deliver WP1 outcomes - May 8th 2019

This slide deck was presented in the webinar on May 8th 2018 to support the delivery of Work package 1 outcomes.

If you are viewing this slide deck, we recommend viewing it together with the webinar recording in order to get a better understanding and context to the visuals presented. For more detailed information and analysis, please check the full reports of this work - links in the end of this slide deck.

If you are using any of the available material in your own work, please reference to it as IMI-Paradigm - Work package 1 outcomes webinar, May 8th 2019 (available at https://imi-paradigm.eu/project-deliverables/).
Presenting speakers and the core team

Work package co-leads

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Online survey analysis &
Modified Delphi methodology

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Focus group consultations

Ana Diaz
Alzheimer Europe

Begonya Nafria
Fundació Sant Joan de Déu

Elisa Ferrer
EURORDIS

Neil Bertelsen
HTAi
Agenda today

12:00   Welcome
12:05   Short introduction to PARADIGM
12:15   Survey and focus group results
12:30   Delphi results
12:45   Questions & discussion
Key Focus

WHAT AND WHY
Multi-stakeholder collaboration to drive meaningful and systematic patient engagement
A collaboration for enhanced patient engagement

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

Advancing meaningful patient engagement (PE) in the life cycle of medicines for better health outcomes

Fueled by Co-creation

European IMI and EFPIA co-funded 30 month project

Multiple stakeholders build benefits for all players
PARADIGM’s definition of patient engagement

The effective and active collaboration
of patients, patient advocates, patient representatives and/or carers
in the processes and decisions within the medicine lifecycle,
along with all other relevant stakeholders when appropriate.
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A Distinct Voice In the Patient Engagement Landscape

Mission
Contribute to a sustainable framework that enables meaningful patient engagement (PE) and demonstrates ‘return on engagement’ for all players

Objectives
Develop processes and tools for these three points in the medicines lifecycle
Develop a sustainability roadmap for patient engagement
## Rationale Behind Selecting Specific Points in the Medicines Lifecycle

### Research and priority setting
- Patients’ perspectives are largely untapped in the research priority setting
- Input on unmet medical needs critical for prioritization
- Patients’ perspective required for determination of value proposition

### Design of clinical trials
- Building block of development necessitates routine patient input
- Voice of vulnerable and unaffiliated patients not yet integrated
- New insights to support generation of standard processes

### Early dialogues with regulators and HTA bodies
- Patients’ input not regularly sought
- Patient perspectives critical to inform access decisions
- Guide generation of standardized processes
Seven Distinct Work Packages to Deliver Specific Outputs

1. Defining stakeholders’ preferences, needs and expectations
2. Assessment of practices and process
3. Co-designed recommendations and resources for patient engagement
4. Development of metrics for monitoring and evaluation
5. Sustainability strategy
6. Dissemination and engagement
7. Project coordination and management
WP1 – Stakeholders’ Needs, Preferences and Expectations Survey and Focus Group Consultations

Results and Key Findings
Objectives of WP1

- **Understand stakeholders’ expectations, needs and preferences** for meaningful PE
- **Involve all relevant stakeholders** who are part of the medicines development process

<table>
<thead>
<tr>
<th>Stakeholders</th>
<th>Healthcare Professionals</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient community</td>
<td></td>
</tr>
<tr>
<td>(patients and their carers, patient advocates and patient organisations)</td>
<td></td>
</tr>
<tr>
<td>Academics</td>
<td>Regulators</td>
</tr>
<tr>
<td>Research Funders</td>
<td>Health Technology Assessment (HTA) bodies</td>
</tr>
<tr>
<td>Policymakers</td>
<td>Payers</td>
</tr>
<tr>
<td>Industry</td>
<td></td>
</tr>
</tbody>
</table>
Reaching objectives in 2 stages

**Stage 1**

The current landscape

- Online survey
- Focus group consultations:
  - People with dementia (Alzheimer Europe)
  - Young people (Fundació Sant Joan de Déu)
  - Rare disease patients (Eurordis)
  - Health Technology Assessment bodies (HTAi)

**Stage 2**

Delphi process

- Prioritise the needs and expectations from the survey (with the 3 expert panel groups)
- Co-create a set of criteria (based on the prioritised expectations) to assess PE practices and processes
Results from the survey and focus group consultations

**Survey**
- 372 English language responses
- 169 non-English language responses
  - 13 non-English languages
- 48 countries

**Focus group consultations**
- 4 face-to-face consultations conducted from June – October 2018
- 11 people with dementia and 10 carers
- 14 young people between 15-18 years old
- 9 patient representatives from rare disease consultation
- 11 representatives from HTA agencies

![Respondents by Country (%)](image-url)
## All respondents

### Stakeholders

<table>
<thead>
<tr>
<th>Stakeholder</th>
<th>Count</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient community (Patients (including carers), Patient advocates, Patient organisations)</td>
<td>200 (76/124)</td>
<td>37%</td>
</tr>
<tr>
<td>Industry</td>
<td>178</td>
<td>32.9%</td>
</tr>
<tr>
<td>Research and Academia</td>
<td>56</td>
<td>10.4%</td>
</tr>
<tr>
<td>Healthcare Professionals (HCP)</td>
<td>32</td>
<td>6%</td>
</tr>
<tr>
<td>Health Technology Assessment bodies (HTA)</td>
<td>20</td>
<td>4%</td>
</tr>
<tr>
<td>Regulator or Policymaker</td>
<td>21</td>
<td>3.9%</td>
</tr>
<tr>
<td>Research funder</td>
<td>4</td>
<td>0.7%</td>
</tr>
<tr>
<td>Payer</td>
<td>1</td>
<td>0.2%</td>
</tr>
<tr>
<td>Other</td>
<td>29</td>
<td>5.4%</td>
</tr>
<tr>
<td><strong>ALL</strong></td>
<td>541</td>
<td></td>
</tr>
</tbody>
</table>
Overall key findings

The current perception of PE is low, yet ideal expectations are high.
Overall key findings

- Industry, patient advocates and organisations, research and academia respondents had the most PE experience.

- Regulators or policymakers, HTA bodies and research funders had the lowest reported PE experience.

<table>
<thead>
<tr>
<th>Overall previous PE experience by stakeholder group</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients (including Carers)</td>
<td>428</td>
</tr>
<tr>
<td>Patient Advocates and Patient Organisations</td>
<td>1129</td>
</tr>
<tr>
<td>Research and Academia</td>
<td>527</td>
</tr>
<tr>
<td>Healthcare Professional</td>
<td>375</td>
</tr>
<tr>
<td>Biotechnology/Pharmaceutical/Medical Technology</td>
<td>1458</td>
</tr>
<tr>
<td>Regulator or Policymaker</td>
<td>110</td>
</tr>
<tr>
<td>Health Technology Assessment (HTA) bodies</td>
<td>140</td>
</tr>
<tr>
<td>Research Funder</td>
<td>55</td>
</tr>
<tr>
<td>Other</td>
<td>284</td>
</tr>
</tbody>
</table>
Previous PE with the patient community

Research Priority Setting

Stakeholder group
- Research and Academia
- Healthcare Professional
- Biotechnology/Pharmaceutical/Medical Technology Industry
- Regulator or Policymaker
- Health Technology Assessment
- Research Funder
- Other

Decision Making Processes with Patients, Patient Advocates or Patient Organizations
Previous PE with the patient community
Previous PE with the patient community
Previous challenges with stakeholders in PE activities

Challenges experienced with a stakeholder group (Total Respondents)

- Patient input was not part of decision making
- Lack of shared vision/ goals with other stakeholders
- Communications were not clear and open
- Lack of openness to have patient input
- Delays in activities due to bureaucratic processes (e.g. Contacts, IP, etc.)

Legend:
- Patients, patient advocates or patient organisations
- Research and academia
- Healthcare professional
- Policymaker or regulator
- Research funder
- Pharma/ biotech/ med tech industry
- Health Technology Assessments (HTA)
- Payer
Desired outcomes of PE at the Research Priority Setting

The three most desired outcomes of patient engagement in the research priority setting? (Total Respondents)

- When patients' needs are leading in the research agenda: 218
- When all parties come to agreement early in the process: 110
- When patients also have a say in what research gets funded: 113
- When it results in new insights and new perspectives for policy makers and regulators and research funders: 214
- When researchers get better insight in the patients' journey: 188
- When it results in mutual learning: 121
- When patients receive feedback about the impact of their engagement: 105
- Other: 17

Counts (n)
Desired outcomes of PE at the Clinical Trial Design Stage

What are the three most desired outcomes of patient engagement in clinical trials? (Total Respondents)

Counts (n)

- When information is better communicated to patients (96)
- When patients can share their experiences and increase knowledge of the clinician (108)
- When it results in more patient-relevant outcomes for the clinical trial (267)
- When it improves recruitment (84)
- When it improves diversity in recruitment (33)
- When it leads to reduced drop-out rate (49)
- When it leads to better compliance (53)
- When it leads to fewer protocol amendments (34)
- When it leads to shorter timelines of trials (95)
- When it leads to an earlier stop of unsuccessful research (61)
- When it leads to higher patient satisfaction during the trial (106)
- When patients receive feedback about the impact of their engagement (87)
- Other (13)
Desired outcomes of PE at the Early Dialogues Stage

What are the three most desired outcomes of patient engagement in medicines licensing and Health Technology Assessment (HTA)? (Total Respondents)
More effective PE

Which of the following would you need to do more effective patient engagement?
(Total Respondents)
## Increasing PE activities for the patient community

<table>
<thead>
<tr>
<th>Respondent group</th>
<th>Type of support needed</th>
<th>Counts (n/total)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients</td>
<td>Person/group with in-depth knowledge in the area of the planned engagement</td>
<td>28/45</td>
</tr>
<tr>
<td>Patient advocates and organisations</td>
<td>Stakeholders responsible for the PE activity I (we) want to engage in</td>
<td>61/88</td>
</tr>
</tbody>
</table>
Patient organisations and participation in PE

How prepared is your organisation to actively participate in patient engagement in terms of the following? (Patient Organisations)

<table>
<thead>
<tr>
<th>Stakeholder group</th>
<th>Not prepared and don't need support</th>
<th>Not prepared and need support</th>
<th>Prepared but need support still</th>
<th>Prepared and don't need support</th>
</tr>
</thead>
<tbody>
<tr>
<td>Internal Processes</td>
<td>2</td>
<td>14</td>
<td>24</td>
<td>48</td>
</tr>
<tr>
<td>Knowledge (information and expertise)</td>
<td>1</td>
<td>9</td>
<td>22</td>
<td>56</td>
</tr>
<tr>
<td>Human resources</td>
<td>5</td>
<td>32</td>
<td>39</td>
<td>56</td>
</tr>
<tr>
<td>Financial resources</td>
<td>3</td>
<td>12</td>
<td>39</td>
<td>42</td>
</tr>
<tr>
<td>Managing competing interests</td>
<td>2</td>
<td>8</td>
<td>35</td>
<td>42</td>
</tr>
<tr>
<td>Setting priorities in your patient engagement strategy</td>
<td>1</td>
<td>17</td>
<td>103</td>
<td>43</td>
</tr>
</tbody>
</table>

Counts (n)
A dedicated PE function within the organization

The majority of (n=99/130) industry respondents reported a dedicated function in their organisation.
A dedicated PE function within the organization

Less than half (n=12/25) of regulators, policymakers, HTA bodies, research funders and payers reported a dedicated function

How are patients or their representatives involved in your work? (Regulators or policymakers, Health Technology Assessment (HTA) bodies, Research funders and Payers)

- Setting research priorities
- Designing clinical trials
- Early dialogues with regulators and/or HTA bodies

Counts (n):

- Deciding: 7
- Consulting: 6
- Informing: 3
- Not involved: 13

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Measuring the impact of PE

<table>
<thead>
<tr>
<th>Respondent Group</th>
<th>Do <strong>NOT</strong> have metrics or methods to measure impact of PE (n/total)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Industry</td>
<td>103/130</td>
</tr>
<tr>
<td>Regulators, policymakers, HTA, research funders, payers and healthcare professionals</td>
<td>11/25</td>
</tr>
</tbody>
</table>
Standard Operating Procedures (SOP)

Have you established a Standard Operating Procedure or other guidance on interactions with patients/patient organisations in medicines development?

- **Yes, and it is used in practice**: 67 ( Pharma/biotech/med tech industry: 7, Healthcare Professionals: 1 )
- **Yes, but it is not used in practice**: 12 ( Pharma/biotech/med tech industry: 1, Healthcare Professionals: 1 )
- **No, but it is not needed**: 6 ( Pharma/biotech/med tech industry: 6, Healthcare Professionals: 6 )
- **No, and it should be established**: 42 ( Pharma/biotech/med tech industry: 11, Healthcare Professionals: 31 )
Focus group findings

<table>
<thead>
<tr>
<th>Focus Group Consultations - Patient groups and more meaningful PE</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>What to consider</strong></td>
</tr>
<tr>
<td>Voice of the person with the condition</td>
</tr>
<tr>
<td>Diversity of patients involved</td>
</tr>
<tr>
<td>Raising awareness of PE opportunities</td>
</tr>
</tbody>
</table>
**Focus Group Findings**

**Focus Group Consultations - HTA and Early Dialogues**

<table>
<thead>
<tr>
<th>Common challenges of PE</th>
<th>Types of tools, resources, guidance needed</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients are unable to make objective inputs</td>
<td>Patient recruitment processes</td>
</tr>
<tr>
<td>Difficulties finding patients (appropriate profile and capacity)</td>
<td>Patient interview guidance</td>
</tr>
<tr>
<td>Details are too complex for patients to engage with</td>
<td>Minimum standards framework</td>
</tr>
<tr>
<td>Conflicts of interest between patient organisations and industry</td>
<td>Rationale for PE in the Early Dialogues stage</td>
</tr>
<tr>
<td>Lack of internal resources (time, financial)</td>
<td></td>
</tr>
<tr>
<td>Lack of clarity to engage patients at the Early Dialogues stage</td>
<td></td>
</tr>
</tbody>
</table>
Conclusions

• PE is crucial and valuable throughout the medicines development process

• Desire for more engagement with more stakeholders and in a more effective way (via metrics, impact, outcomes)

• The patient community had a different take on the greatest challenges to PE compared to the other stakeholders

• Integration of the PE activity into the decision-making process and reflected in the outcomes of the activity

• There is still need for more support to engage in a PE activity in two ways - *what to do* and *how to do it*
Reaching objectives in 2 stages

Stage 1
The current landscape

• Online survey
• Focus group consultations:
  – People with dementia
  – Young people
  – Rare disease patients
  – Health Technology Assessment bodies

Stage 2
Delphi process

• Prioritise the needs and expectations from the survey (with the 3 expert panel groups)
• Co-create a set of criteria (based on the prioritised expectations) to assess PE practices and processes
Delphi processes for co-prioritisation

Development and validation of a framework for prioritisation questionnaires

Survey & focus group results

Literature review

Background data

Meeting in person to reach final consensus of the key criteria and their definition

Setting research priorities

Designing clinical trials

Early dialogues with regulatory and HTA

Expert panels go through prioritisation exercise first online (2 rounds)

Criteria to assess how patient engagement expectations are met
## Representative expert groups

<table>
<thead>
<tr>
<th>Participants</th>
<th>Initially</th>
<th>Round 1</th>
<th>Round 2</th>
<th>Round 3 F2F</th>
<th>Profiles in the F2F meeting</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>DELPHI</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Setting research priorities</td>
<td>24</td>
<td>20</td>
<td>16</td>
<td>10</td>
<td>Research funders (public and/or private), Regulator, Academic, Healthcare professionals, Patients, Pharma company</td>
</tr>
<tr>
<td>Design of clinical trials</td>
<td>31</td>
<td>29</td>
<td>26</td>
<td>18</td>
<td>Researcher funder, Healthcare professionals, HTA, Regulators, Patients, Pharma company, Academic/Researcher</td>
</tr>
<tr>
<td>Early Dialogues with HTA and Regulators</td>
<td>26</td>
<td>20</td>
<td>18</td>
<td>12</td>
<td>Research funders (public and/or private), Regulator, Academic, Healthcare professionals, Patients, Pharma company</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>81</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
## Results: Criteria to assess patient engagement practices

<table>
<thead>
<tr>
<th>Setting Research Priorities</th>
<th>Designing Clinical Trials</th>
<th>Early Dialogues with Regulators and HTA</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>CRITERIA</strong></td>
<td><strong>WEIGHT</strong></td>
<td><strong>CRITERIA</strong></td>
</tr>
<tr>
<td>Key Elements of Practice Design</td>
<td>19.5</td>
<td>Aim and objectives</td>
</tr>
<tr>
<td>Resources</td>
<td>16</td>
<td>Patient engagement impact</td>
</tr>
<tr>
<td>Evaluation of the PE Practice in Setting Research Priority</td>
<td>12</td>
<td>Target patients involved</td>
</tr>
<tr>
<td>Capacity Building</td>
<td>12</td>
<td>Legal and ethical consideration</td>
</tr>
<tr>
<td>Patient Engagement Impact</td>
<td>11.5</td>
<td>Involvement and participation</td>
</tr>
<tr>
<td>Involvement and Participation</td>
<td>11</td>
<td>Resources</td>
</tr>
<tr>
<td>Code of Conduct</td>
<td>10</td>
<td>Capacity building</td>
</tr>
<tr>
<td>Sustainability</td>
<td>8</td>
<td>Evaluation of the PE practice in the Design of Clinical Trials</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>100</strong></td>
<td><strong>TOTAL</strong></td>
</tr>
</tbody>
</table>
What’s next for PARADIGM?

1. These results are used in the creation of a gap analysis to determine which needs and expectations are not met at the moment

2. Tools created to build capacity and capabilities to do more patient engagement
Acknowledgements

Thank you to:

- PARADIGM partners and International Liaison Group,
- survey respondents,
- focus group participants,
- Delphi panel experts,
- and all PARADIGM supporters for spreading the word about this important work!
Questions & discussion
Thank you!
Useful resources

Official report on the results of the online survey and focus group consultations

- In-depth report from consultation with people living with dementia by Alzheimer Europe
  
- In-depth report from consultation with children and young people by FSJD
  
- In-depth report from consultation with rare disease patients by EURORDIS
  
- In-depth report from consultation with HTA community by HTAi

Official report on the criteria to assess how patient engagement expectations are met


Will be shared on www.imi-paradigm.eu once available.