Patient-Powered Research Networks for Relative Effectiveness Research

Results of a Survey Among Healthcare Decision Makers and Key Opinion Leaders

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Abbreviations

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<tr>
<th>Abbreviation</th>
<th>Description</th>
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<tbody>
<tr>
<td>CER</td>
<td>Comparative Effectiveness Research</td>
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<td>EMR</td>
<td>Electronic Medical Record</td>
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<td>ER</td>
<td>Effectiveness Research</td>
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<tr>
<td>HTA</td>
<td>Health Technology Assessment</td>
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<tr>
<td>ICD</td>
<td>International Classification of Diseases</td>
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<tr>
<td>IMI</td>
<td>Innovative Medicines Initiative</td>
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<tr>
<td>KOL</td>
<td>Key Opinion Leader</td>
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<td>PCOR</td>
<td>Patient-Centered Outcomes Research</td>
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<td>PCORI</td>
<td>Patient-Centered Outcomes Research Institute</td>
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<td>PCORnet</td>
<td>Patient-Centered Outcomes Research Network</td>
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<tr>
<td>PI</td>
<td>Principal Investigator</td>
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<tr>
<td>PPRN</td>
<td>Patient-Powered Research Network</td>
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<tr>
<td>PRO</td>
<td>Patient-Reported Outcome</td>
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<td>QoL</td>
<td>Quality of Life</td>
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## Executive Summary – Overview

<table>
<thead>
<tr>
<th>Awareness</th>
<th>All key opinion leaders had heard of patient-powered research networks (PPRNs). Conversely few healthcare decision makers had awareness of PPRNs. This suggests an opportunity is available to increase awareness among healthcare decisions makers about the existence and uses of PPRNs.</th>
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<tr>
<td>Usefulness</td>
<td>Most participants thought that PPRNs could be useful for comparative effectiveness research (CER) and were able to provide a variety of advantages. However, participants also cited limitations of using PPRNs for CER. Throughout the survey participants were not entirely consistent in their views of PPRNs, suggesting there is currently a degree of ambiguity towards them.</td>
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<tr>
<td>Acceptability</td>
<td>Most healthcare decision makers reported that they thought the use of effectiveness evidence from PPRNs for making healthcare decisions was acceptable. However, when asked to justify this many also provided caveats and limitations.</td>
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<tr>
<td>Obstacles</td>
<td>Some key obstacles cited by respondents were: the burden around ethics and regulations, systematic/standardised data collection and validation of self-reported data. These obstacles need to be addressed in order to make PPRNs more useful for the collection of data for CER.</td>
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<tr>
<td>PROs vs EMR</td>
<td>Both respondent types thought the PPRNs containing electronic medical records (EMRs) and patient-reported outcomes (PROs) were most useful in CER. However, the desktop research revealed that few of these exist (36% of identified PPRNs fall into this category). Therefore this also needs to be addressed by enabling more PPRNs to have access to EMRs.</td>
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</table>
Executive Summary – Recommendations

1 Increase PPRN usage by decision makers

- Most healthcare decision makers were positive about the use of PPRNs for CER, however, as seen in our desktop research (separately published) most PPRNs appear to not yet be in a position to produce strong CER.
- Healthcare decision makers do, however, greatly value the data PPRNs already have – that which relates to the patients subjective experience of their disease and treatment
- Hence, healthcare decision makers could currently be encouraged to make use of existing PPRN data in order to allow them access the patients subjective point of view
- This access in turn would increase their familiarity with and positive view of PPRNs

2 Increase EMR access

- A strong next step in PPRN development has been found to be an increase in the number of PPRNs containing both EMR and patient reported outcome data
- Obtaining EMR access for PPRNs would in part be aided by advocacy among healthcare decision makers

3 Usage could help with access

- Hence the above suggested action of increasing PPRN usage among healthcare decision makers now could lead in the future to decision maker advocacy which in turn could help in obtaining EMR access. Having both EMR and patient reported outcomes would then push PPRNs closer to a point where they could generate strong CER

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Background
Background

Patient-powered Research Networks provide a novel source of real-world data, potentially valuable for comparative effectiveness research

- Patient-powered Research Networks (PPRNs) are online platforms where patients share their health data with other people or organisations of their choice, with health data coming from either the patients directly (e.g. PROs) or their electronic medical records, or both

- The usefulness and acceptability of PPRN data in Comparative Effectiveness Research (CER) is unclear

- As part of IMI’s GetReal Project, this report presents findings of a survey among relevant key opinion leaders and healthcare decision makers that aimed to assess the usefulness and acceptability of PPRNs in CER as well as their future role and their impact on patients
Objectives
Objectives
Objectives of the survey among healthcare decision makers and key opinion leaders to assess the usefulness and acceptability of PPRNs for CER

This aim will be achieved through the following study objectives:

• Characterise the potential **usefulness of PPRNs** (in particular, PCORnet PPRNs) to regulators, HTAs and payers for evaluating the relative effectiveness of products
  – Identify any **disease areas** for which PPRNs are particularly useful
  – Understand the **limitations** and practical feasibility of using PPRNs for relative effectiveness studies

• Assess the level of **acceptability** of PPRNs to regulators, HTAs and payers for measuring relative effectiveness

• Characterise the potential **future role of PPRNs** in effectiveness research (~10 year time horizon) and any implications for effectiveness research in general in Europe

• Understand the potential **impact of PPRNs on patients** and patients’ views of PPRNs
Approach by Project Objective

This report presents the results of the KOL and healthcare decision maker interviews

1. Classification of PPRNs
   - Literature review*
   - Desktop research*

2. Usefulness of PPRNs
   - Literature review*
   - Desktop research*

3. Acceptability of PPRNs
   - KOL and healthcare decision maker interviews

4. Future role of PPRNs
   - KOL and healthcare decision maker interviews

5. Impact on patients
   - KOL and healthcare decision maker interviews

*Results of the literature review and desktop research are presented in a separate report
Methods
Overview

Process and participants

• All of the interviews were conducted by phone, the first was on the 12 April 2016 and
  the last was on the 14 June 2016

• Interviewees were KOLs/stakeholders with an interest in PPRNs and/or CER or
  healthcare decision makers working as payers or at regulatory agencies and HTA
  bodies

• Participants were sent definitions relating to PPRNs and relative effectiveness prior to
  the interview. These definitions were also read out at the beginning of the interview

• The survey referred to PPRNs in general, rather than looking at PCORI and non-PCORI
  PPRNs separately. The survey was designed as such because many of the respondents
  would have been unable to answer questions referring to specific PPRNs or PCORI
Methods for Survey Among Healthcare Decision Makers and Key Opinion Leaders

- Potential participants were identified from desktop research or from existing contacts
- Healthcare decision makers and KOLs/other stakeholder were recruited by email and by phone

Summary transcriptions of interviews were analysed in 3 different ways:
- Exceptional paraphrased comments were highlighted to be included in the final report
- Answers were assigned relevant categories and grouped
- Categories were collated and summarised as counts or % where appropriate

The questionnaire was developed by IMS and the GetReal team and was based on the objectives outlined in the proposal
- All interviews were conducted by phone
- Interviews were scheduled to last for 30 minutes

If participants agreed to being recorded, the entire interview was recorded and referred back to later
- Notes were taken to summarise and record participants’ responses
- Comments provided are not full quotes but summarised transcriptions

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Recruitment Overview
The two respondent groups had similar response rates

- All participants were sent a first invite email. This email outlined the study's purpose and structure and informed participants that their responses would be reported anonymously, that they would receive an incentive for taking part and that they should contact us for further information.

- Those participants who did not respond to the first invite were then sent a reminder email.

- Some contacts did not respond to either email. If we had phone numbers for these persons, they were then contacted by telephone and further emails were sent when

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<th></th>
<th>KOLs</th>
<th>Decision Makers</th>
<th>Total</th>
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<tbody>
<tr>
<td>Invited</td>
<td>149</td>
<td>133</td>
<td>282</td>
</tr>
<tr>
<td>Participated</td>
<td>21</td>
<td>19</td>
<td>40</td>
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<tr>
<td>Response rate</td>
<td>14.09%</td>
<td>14.29%</td>
<td>14.19%</td>
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Participants Overview

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<tr>
<th>Healthcare decision makers</th>
<th>KOLs/other stakeholders</th>
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<tbody>
<tr>
<td>• Identified through collaboration with the IMS Market Access team and via desktop research</td>
<td>• KOLs were identified through the GetReal literature review and desktop research and were also suggested through the internal IMS network</td>
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<tr>
<td>• Focus on decision makers who would evaluate treatments through reviewing available evidence</td>
<td>• This group included individuals involved directly with PPRNs or PCORI and others with experience working with real-world data</td>
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<td>• Some of this group were working at a regional level (e.g. within the NHS) and some at a national level (e.g. NICE)</td>
<td>• Based mostly in the US with some respondents coming from the UK, Canada, Belgium and Germany</td>
</tr>
<tr>
<td>• All of this group were based in the UK</td>
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<table>
<thead>
<tr>
<th>General</th>
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<tr>
<td>• Participants were invited to participate in the survey via email and by phone</td>
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<tr>
<td>• Interviews were conducted by phone</td>
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<tr>
<td>• They are to remain anonymous i.e. their identification will not be revealed to project sponsors or linked to their results in the final report</td>
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<tr>
<td>• A small financial incentive was provided to them (or donated to charity) for their participation</td>
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Questionnaire Development

The questionnaire was developed and piloted internally

- The questionnaire was developed by IMS and the GetReal team and was based on the objectives of the survey.

- Once the initial questionnaire was agreed upon, it was piloted among five relevant individuals and small adjustments were made to the final version based on their feedback.

- The questionnaire contained 17 questions (some with more than one part):
  - Five of these questions contained closed-ended elements, i.e., allowed the respondent to give a yes/no response, provide ratings on a numeric scale or a selection of answers to choose from.
  - 11 of these questions contained open-ended elements, i.e., the respondent was able to freely give their views or opinions.

- Five of the questions were specifically for KOLs only and two only for healthcare decision makers.

- Participants were read out definitions relating to effectiveness research, comparative effectiveness research and patient-powered research networks before being asked any questions.
Definitions Provided for Participants

There definitions were read out before the first question and were read again where required during the interview

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<th>Effectiveness research</th>
<th>Comparative effectiveness research</th>
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<tr>
<td>• Is the evaluation of a medical treatment’s performance in real-world settings (i.e. during normal healthcare practice) as opposed to the evaluation of a treatment in the controlled setting of a clinical trial</td>
<td>• Is about comparing the effect of one medical treatment against one or more other treatments in real-world settings</td>
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Patient-Powered Research Networks (PPRNs)

• Are online platforms, operated and governed by patients and their advocacy organisation, clinicians and clinical research partners. They collect medical data from patients directly (i.e. self-reported data including PROs) and/or from patients’ electronic medical records (which the patients typically upload and control access to) for the purpose of accelerating effectiveness research. Examples of information which may be collected by PPRNs are demographics, diagnosis, medication information and symptoms
Results – PPRN Definition
All KOLs but Only a Few Healthcare Decision Makers Had Heard of PPRNs

Participants’ awareness of PPRNs or similar entities following reading of PPRN definition

Participants were first read the PPRN definition on slide 17 before answering this question

All participants N= 40

Q2. Had you heard about patient-powered research networks or networks which fit the definition we have given before we contacted you about this survey?
The Majority of KOLs Were Positive about the PPRN Definition Provided

The PPRN definition provided was frequently described as accurate and comprehensive.

- **Positive about definition**: 71%
- **Unsure/mixed**: 24%
- **Negative about definition**: 5%

KOLs/other stakeholders only N= 21

Q1. What do you think about the patient-powered research networks definition we have provided? If respondent answers briefly with ‘it is okay’ prompt them by asking ‘Is there anything we could do to improve it?’

Top Mentions

- Accurate/comprehensive: 50%
- Describes our platform/work I've done: 18%

Top Mentions

- Not necessarily online: 75%
- Difficult to link EMR: 14%

Top Mentions

- Involves patients only: 50%
- Not dedicated to effectiveness research: 50%

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## Responses regarding PPRN Definition: Key Highlights

Comments on the definition provided for PPRNs

<table>
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<tr>
<th>Response category</th>
<th>Response</th>
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<tbody>
<tr>
<td>Accurate/comprehensive (n=11)</td>
<td>“It is helpful because it defines <strong>governance, purpose and activities</strong>, so I think it’s a good definition in being specific in that way”</td>
</tr>
<tr>
<td>Involves patients only (n=1)</td>
<td>“Wrong, and limited, I think PPRNs, if they are going to be transformational in biomedical research, <strong>would be patient powered</strong>. Not clinicians, not anyone else in research realm, we already have a lot of that, we actually have already had PPRNs that are actually run by patients, so I don’t know why we are saying they are run by clinicians. I don’t think a PPRN could be run by a clinician or a researcher”</td>
</tr>
<tr>
<td>Not necessarily online platforms (n=4)</td>
<td>“Guess the <strong>name doesn’t dictate that they have to be online</strong>, could involve any existing research these organisations carry out as well (suggestion for improvement) Digital patient powered networks – if really looking at digital platforms, name needs to capture that. If clinicians and clinical research partners are involved that could involve any clinical research”</td>
</tr>
</tbody>
</table>

Q1. What do you think about the patient-powered research networks definition we have provided? If respondent answers briefly with ‘it is okay’ prompt them by asking ‘Is there anything we could do to improve it?’
Results – Usefulness of PPRNs for Effectiveness Research
Most Respondents Thought PPRNs Could Be Useful for Conducting Effectiveness Research

None of the participants reported that they thought PPRNs could not be useful for conducting ER

Although most respondents reported that they thought PPRNs could be useful for conducting effectiveness research, when asked why, many participants also reported limitations as well as the advantages of using PPRNs for this purpose – this implies that even when generally positive about PPRNs, respondents had the limitations top of mind.

Therefore, the responses to Q4 ‘Why do you think this (PPRNs useful/not useful for Effectiveness Research)?’ were divided into positive and negative comments and combined with responses from questions 5 (PPRN advantages - ER) and 6 (PPRN limitations - ER)

Q3. Do you think patient-powered research networks (PPRNs) could be useful for conducting effectiveness research?
KOLs and Healthcare Decision Makers Often Cited Similar Advantages of Using PPRNs

A commonly cited advantage of using PPRNs for effectiveness research was that they involve patients’ perspectives

Advantages of using PPRNs for Effectiveness Research

- Patient perspective and involvement
- Overall useful/adds additional information
- Real-world data
- Sample/data more representative than RCTs
- Large amounts of data
- Enables study recruitment
- Better outcomes for patients
- Continue to study treatments after RCTs/longitudinal data
- Involves QoL
- Cost effective/efficient
- Can investigate sub-groups of patients
- Real-time data collection

Q5. Could you provide your view of one or more of the advantages of using patient-powered research networks (PPRNs) for comparative effectiveness research?
Advantages of Using PPRNs for Effectiveness Research

Key responses

<table>
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<tr>
<th>Response category</th>
<th>Response</th>
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<tbody>
<tr>
<td>Patient perspective/Involvement</td>
<td>“PPRN voices can be really helpful there. Having someone speak about the impact the disease had on their own life can be really helpful and powerful for people who don’t have that insight or speak regularly to researchers in hospital and health systems. They (researchers) don’t get that perspective that often” – KOL</td>
</tr>
<tr>
<td>Overall useful/additional info</td>
<td>“It’s unique information, it’s required on a lot of issues, a bit less comparable but may be more efficient, convenient for patients and may be improving QOL of patients. That information is just not available [in the current data]” – KOL</td>
</tr>
<tr>
<td>Real World Data</td>
<td>“I think there needs to be a combination of PPRNs and clinical trials. Using them on their own doesn’t give the whole picture. How medicines should be used in practice for people who don’t look like the people who are involved in clinical trials, and that’s where the power of real world data comes into use” – Decision Maker</td>
</tr>
<tr>
<td>Sample more representative</td>
<td>“In clinical trials you don’t see a full representation of society, whereas when open up outside the clinical trial population, you can go to broader public meaning, you don’t have exclusion inclusion criteria, so you can go much deeper in real life situation to assess effectiveness” – KOL</td>
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Q5. Could you provide your view of one or more of the advantages of using patient-powered research networks (PPRNs) for comparative effectiveness research?
A High Proportion of Respondents Felt That Bias May Be a Limitation of Using PPRNs for Effectiveness Research

Limitations of using PPRNs for Effectiveness Research

- Self reported data may be biased or inconsistent
- The patient population may be biased
- Low health literacy/IT abilities
- Recruitment/practical difficulties
- Organisations/clinicians involved may introduce bias
- Variation within PPRNs/no standardization
- Messy/low quality data
- Regulators may not value/use this information
- Ethical issues/concerns e.g. privacy
- Issues with combining data types
- Cannot use for all diseases e.g. asymptomatic
- Not as useful as RCTs
- Varied evaluation methods

“I guess there are biases attached to using PPRNs in that we know from research that biases exist when you use real-world, pragmatic, observational data from databases. So we need to make sure checks and measures are in place to minimize biases, just like we do in RCT data or clinical trial data” – Decision Maker

All participants
N= 40
Q6. Could you provide your view of one or more limitations of using patient-powered research networks (PPRNs) for comparative effectiveness research?

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Limitations of Using PPRNs for Effectiveness Research

### Key responses

<table>
<thead>
<tr>
<th>Response category</th>
<th>Response</th>
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<tbody>
<tr>
<td>Self reported data may be biased/inconsistent (n=21)</td>
<td>“Self reporting doesn’t necessarily mean consistent reporting, so the robustness of the data collected could be questioned” – Decision Maker</td>
</tr>
<tr>
<td>Patient population may be biased (n=16)</td>
<td>“Accessing patients in an equitable manner, and ensuring data gathered is representative of the whole population, not just those who are fit and well enough to access online platforms.” – Decision Maker</td>
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</table>
| Regulators/stakeholders may not value or use the info (n=4) | “Subject to being hijacked or biased by funders, there can be conflicts of interest with funders or manufacturers with pharma in particular” – KOL  
“Clinical research partners and pharmaceutical partners have a conflict of interest in that they want to get their product used. We need to be cautious about their involvement in terms of robustness of information provided in contrast with clinical data provided” – Decision Maker |

Q6. Could you provide your view of one or more limitations of using patient-powered research networks (PPRNs) for comparative effectiveness research?
Results – Acceptability of Using PPRNs for Effectiveness Research
Acceptability of Using PPRNs for Effectiveness Research within the Remit of Healthcare Decision Making

Most healthcare decision makers felt that effectiveness research based on PPRN data is acceptable for making healthcare decisions.

“PROs are rarely used and can’t think of examples where they’ve influenced decisions made by committee, low on 2 – not because committees are prejudiced against them, just quality of data generally not good enough”

“Depends on which therapeutic area, what condition, and what outcomes are measured, in some cases it would be acceptable e.g. Crohn’s, respiratory, in other cases it won’t be acceptable”

“We need to get people’s views as to effectiveness of treatment, rather than just clinicians. If we don’t listen to them, how will we ever optimize their care?”

Acceptability of Using PPRNs for Effectiveness Research within the Remit of Healthcare Decision Making

Q10. To what extent do you think the use of patient-powered research networks (PPRNs) for conducting effectiveness research is acceptable for use in your professional role for making healthcare decisions? Please give your rating on a scale of 1 to 5 where 1 = unacceptable, 3 = neutral and 5 = acceptable

Q11. Why did you give that rating?
Results – Future Role and Obstacles
The majority of KOLs felt PPRNs would have a role in future research in Europe in 5-10 years.

**PPRNs: European role in 5-10 years**

- **Top Mentions**
  - Regulators not using PROs: 29%
  - Funding and support required: 14%
  - If combined with clinical validation: 14%

- **Top Mentions**
  - Need for PROs/RWE in research: 23%
  - Europe already involves patient in healthcare/decision making: 19%

KOLs/other stakeholders only

N= 21

Q12. Do you think PPRNs will have a role in effectiveness research in Europe in 5-10 years time?
Q13. Why do you think this?
Many Respondents Reported Obstacles Related to Ethics and Regulations

Also of interest were the standardisation of data collection and difficulty in validating self-reported information

**Obstacles to be overcome to make PPRNs more useful for effectiveness research**

- Burden around ethics and regulations e.g. privacy, consent
- Systematic/standardised data collection
- Validation of self-reported data
- Quality of PPRN data
- Technology/EMR integration
- Communication and the need for a common language
- Biased sample
- Regulators/researchers awareness of PPRN data
- Funding/sustainability
- Recruiting/engaging patients
- Negative perception of self-report data
- Need to have enough information about patient

“Common language, challenge for people from different walks of life to be able to get together and have meaningful conversations which result in collaborative effort which result in actual changes in how research is done…we don’t know how to bring different voices together, some of it relies on language and translation, help everyone have a common language at starting point to be able to share that information.”

KOLs/other stakeholders only

N= 21

Q14. What obstacles need to be overcome to make patient-powered research networks (PPRNs) more useful as a data source for effectiveness research?
Obstacles to Overcome...Highlights (1 of 2)

Ensure secure systems are in place, ensure systems can't be gamed. If PPRNs are used for CER that goes to NICE and HTA agencies, you have an incentive for unethical people to attempt to systematically gain data to make the product look better, if trust is undermined, the system can't move forward.

Issues regarding anonymity and who owns data and where it sits, and confidentiality and opt in opt out, and nature of what it is possible to record, technologies need to improve before they’re most useful.

Managing expectations of PPRNs will be very important, so everyone has awareness of limitations and benefits of using them.

Greater governance around the ethical conduct, need for more technical advancement around anonymous linkage of data, especially when linking PROs to EMRs and ICD codes from a payer. But I also think attitude and perception, there is often the perception that patients are a second class citizen group and that their data is anecdotal data and messy and adds flavour versus it having rigour and validity.

There is always a balance between the individual and unique person and those things which can be more generalisable, it’s going to be challenging to balance these two things. We need to be sure we’re using objective and valid measures even as we’re allowing the influence of patient perspective in, got to stay within the scientific paradigm.

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Obstacles to Overcome…Highlights (2 of 2)

It would have to be addressed from the get go, from the design of the study. It’s **all about subject recruitment**, making sure you have a **variety of ways of recruitment** to get different types of people and as you are recruiting people, you are not going to limit the people.

**One approach is to have a forum through which PPRNs can interact with traditional comparative effectiveness researchers.**

**Many organisations need to work with each other to make it work, not something that one organisation can work out by itself, and there have to be expert meetings with the statisticians, epidemiologists, the payer, the payee, so yes it has to be team work to solve this.**

The **providers need to have a value of it [PPRNs]**. They need to see useful information coming out of it [PPRNs]. So they **contribute but they get something back**.
Overcoming the Burden around Ethics and Regulations

Respondents' suggestions of how the obstacles can be overcome

“Need to have legal advisors, need to be able to test what you do in a medical setting which will give you feasibility and credibility with your patients and doctors you are talking with...need to provide some specific steps to be able to comply with the regulations and use what you are doing with patients for the objective you want to show”

“...trying to find balance between degree of privacy and expectation that they [patients] will get the best possible therapy which will involve some things which run counter to privacy...”

“Educating people and showing benefit of sharing information, and why it’s useful”

KOLs/other stakeholders only
N= 21

Q15 How would you suggest these obstacles should be overcome?

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Overcoming Data Collection and Self-report Validation Obstacles

Respondents’ suggestions of how the obstacles can be overcome

- “Structure data, have option to donate data to structured data banks which can then be used for research, also important to manage quality of data”

- “Need to come up with standard agreed definitions of what you mean by terms in the same way we did in this interview”

- “…partner with other organisations with databases and run comparison studies with them…publish work on differences and how to understand implications…study about proving patients are who they say they are…partnered with…insurance claims company…”

KOLs/other stakeholders only
N= 21
Q15 How would you suggest these obstacles should be overcome?
**Overcoming Data Quality and Technology Obstacles**

Respondents’ suggestions of how the obstacles can be overcome

- **“People need to become more comfortable with quality of data** from these networks, which will happen with time and more examples of these networks coming forward, and with technological advances, e.g. sensors on smartphones, which will inform networks more cheaply, the level of information you’re able to collect is more valuable.”

- **“Show how PPRNs are valuable, there is misunderstanding about what they can and can’t do, need to understand more about why they are good and what they can offer, think in next 2-3 years this will be shown – lots of demonstration projects to show why PPRNs are good”**

- **“Technologically no – but I’m sure people will come up with ways of making this data easier to use”**

**KOLs/other stakeholders only**

N= 21

Q15 How would you suggest these obstacles should be overcome?

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Overcoming Communication and Sample Bias Obstacles

Respondents’ suggestions of how the obstacles can be overcome

“One thing that has been successful in PCORnet...is having regular interaction between clinical data research networks...and researchers and PPRNs on a regular basis, so there is an interchange at a network level between PPRNs and CDRNs, both in terms of designing a system that allows for PPRN research and the implementation side so once they are established and speaking the same language, having that infrastructure activated so that research can happen across both for traditional researchers and PPRNs, leverage both, that that is key”

“It would have to be addressed from the get go, from the design of the study. It’s all about subject recruitment, making sure you have a variety of ways of recruiting to get different types of people and as you are recruiting people, you are not going to limit the people. ...I would need to set up methodological techniques to quantify the level of bias. I’m really worried about recruitment bias”

KOLs/other stakeholders only
N= 21

Q15 How would you suggest these obstacles should be overcome?
Overcoming Awareness of PPRNs and Funding and Sustainability Obstacles
Respondents’ suggestions of how the obstacles can be overcome

“The providers need to have a value of it [PPRNs]. They need to see useful information coming out of it [PPRNs]. So they contribute but they get something back”

“Sustainability ...most optimistic in ability to overcome...infrastructure developed...substantial investment in building something that can do large scale research, now needs to be used to ask scientific questions...need external funding, but this can come from anywhere... just a matter of making potential sponsors aware that this is an infrastructure that’s ready to be utilised and thereby do high quality science...sustainability question will be answered”

KOLs/other stakeholders only
N= 21
Q15 How would you suggest these obstacles should be overcome?
Overcoming the Obstacle of Recruiting and Engaging Patients

Respondents’ suggestions of how the obstacles can be overcome

“There has to be a **PCORI PPRN strategic plan for how to continue to engage the patients.** Currently PPRNs are all independent, even though they are trying to **work together**, trying to get best practices, there is no formal survey on what is and isn’t working. We really **need to learn from failures** since this is in many ways an innovative and brand new concept. **Shared central resources to implement what is and isn’t working**”
Results – Impact on Patients

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The Impact Respondents Feel PPRNs Will Have on Patient Outcomes

Participants were first asked to think about the impact on outcomes at an individual patient level, then they were asked to consider this at a population level.

Q16. What impact do you think patient-powered research networks (PPRNs) will have on patients in relation to:
Individual patient outcomes
Population-level health outcomes

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Results – Evaluation of PPRN Types
PPRNs Containing EMRs and Self-reported Information Were Rated as Most Useful for Effectiveness Research

There was little variation between ratings from KOLs and healthcare decision makers.

**Mean ratings**

- **Healthcare Decision Makers**
  - EMR alone: 6.0
  - Self-report alone: 6.5
  - EMR and Self-report: 8.5

- **KOLs**
  - EMR alone: 6.0
  - Self-report alone: 6.5
  - EMR and Self-report: 8.5

36% of PPRNs are in this category*

* Results from the GetReal desktop research (separately published)

**Q17. How would you rate a PPRN containing only electronic medical records/only self-reported data/both electronic medical records and self-reported data? Could you rate them for their usefulness for collecting data for treatment effectiveness research on a scale of 1 to 10 where 1 = not useful at all and 10 = very useful**

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Many Decision Makers Felt That EMR Only PPRNs Would Be Factual and Objective
Inconsistent physician reporting was cited as a disadvantage of EMR only PPRNs

**Positive Points about PPRNs containing EMR alone**
- Factual/objective
- Useful for diagnosis, treatments, demographics, test results etc.
- Large quantities of data, can study populations
- Already accessible (positive)
- Unbiased

**Negative Points about PPRNs containing EMR alone**
- Less rich than/lacking self reported information
- Inconsistent physician reporting in EMR
- Unsure if counts as PPRN
- EMR’s not standardised/variation between
- Already accessible - do not need PPRN
Healthcare Decision Makers Cite Less Positives vs KOLs in Relation to Patient Reported Outcome Based PPRNs

Unique values were a leading positive aspect while inconsistent self reporting was a leading negative point.

**Positive** points about PPRNs containing self-reported information alone:
- Adds unique information/value
- See what is important to patients
- Large samples - can look at subgroups/populations
- Collected in real time/longitudinally

**Negative** points about PPRNs containing self-reported information alone:
- Inconsistent self-report
- Missing essential data from EMR
- Difficult to validate
- Patients lack expertise - less accurate/relevant reporting
- Biased sample
- Reporting bias
- Recall bias

**KEY POINT:** The leading negative points here may summarise the barriers to patient self reported PPRNs being taken seriously and would need to be addressed to increase their acceptability. This is especially important when 58% of the PPRNs identified in the desktop research (separately published) are based on patient reported outcomes only. Some of these barriers could be addressed using the solutions to obstacles suggested earlier in this report – suggestions such as educating people about what PPRNs can and cannot do.

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KOLs Frequently Reported That a Strength of EMR and Self-report PPRNs is the Ability to Validate Their Content

There were very few reported negative points about PPRNs containing EMR and self-reported data.

**Positive** points about PPRNs containing both EMR and self-reported data:
- Allows validation of content
- Both perspectives (physician and patient)
- Objective (clinical) and subjective (self-report) information combined
- Gold standard
- Both necessary for effectiveness research

**Negative** points about PPRNs containing EMR and self-reported data:
- Biased sample
- Data quality issues
- Still less useful than RCTs
- Still missing information e.g. health plan/insurance in US
- Difficult to combine/standardise

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Results – Usefulness of PPRNs by Disease Area
The Majority of Participants Reported That All Disease Areas Were Suitable for PPRN Effectiveness Research

The disease areas with the most ‘unsuitable’ and ‘unsure’ responses were mental & behavioural, neurological and cardiovascular.

* As answers given in relation to the pros and cons of using neurological, cardiovascular and cancer disease areas were particularly interesting these have been kept in this section while for the sake of brevity such details for other disease areas have been moved to the appendix.
KOLs More Commonly Thought Disease Areas Were Suitable for ER Using PPRNs vs Healthcare Decision Makers

Q7. For each of the following disease areas, could you say whether you think they are suitable or unsuitable for effectiveness research using data from a PPRN or whether you are unsure?

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Disease Area Focus: Neurological

Negative points related to the dependence on disease severity and limitations due to nature of the disease

**Positive points** about using PPRNs to conduct effectiveness research for **neurological conditions**

- Share information otherwise not collected
- Need more RWD in area
- Lived experience
- Can report at any time
- Existing work in field
- Side effects/adverse events
- Important whether patient thinks treatment works
- Chronic conditions

**Negative points** about using PPRNs to conduct effectiveness research for **neurological conditions**

- Depends on disease or severity
- Nature of disease may mean self-report unreliable
- Need carer/objective observer to report
- Nature of disease may mean patients less likely to use PPRN

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## Reasons Neurological Conditions Are Suitable for ER Using PPRNs

### Key responses

<table>
<thead>
<tr>
<th>Response category</th>
<th>Response</th>
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<tbody>
<tr>
<td>Could include caregiver (n=1)</td>
<td>“Also for caregivers around issues with goal attainment, some of the work I did was looking at Alzheimer’s and broader dementia progression, first it’s about patient driven autonomy, and over time more about patient safety and caregiver burn out, <strong>P in PPRN isn’t just patient but loved ones and caregivers</strong>” – KOL</td>
</tr>
<tr>
<td>Can report at any time (n=3)</td>
<td>“Might be in some cases easier to get them to respond in their own time online rather than at a specific place face to face, challenges with Alzheimer’s depending on who your Alzheimer’s patient is” – KOL</td>
</tr>
<tr>
<td>Lived experience (n=3)</td>
<td>“Same reasons, a <strong>disease for which there is interpreted experience</strong> and there is a need for patient to be engaged and informed. To affect proper treatment becomes so important” – KOL</td>
</tr>
</tbody>
</table>
## Reasons Neurological Conditions Are Unsuitable for ER Using PPRNs

### Key responses

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<thead>
<tr>
<th>Response category</th>
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</tr>
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<tbody>
<tr>
<td>Nature of disease may mean self-report unreliable (n= 10)</td>
<td>“[with] Alzheimer’s, [ER using PPRNs would be applicable] <strong>only in early stages</strong> when they still have memory; [with] MS there are cognitive disturbances as the disease progresses. Parkinson’s also cognitive problems. <strong>Hard to measure, unstable regimes, inaccuracy in recalling</strong>” – Decision Maker</td>
</tr>
<tr>
<td>Need carer/objective observer to report (n=9)</td>
<td>“If a patient’s confused, don’t know what they’re reporting, I guess it <strong>would have to be a carer [reporting], more difficult to get patient perspective</strong> on [these conditions]– Decision Maker</td>
</tr>
<tr>
<td>Depends on disease or severity (n=15)</td>
<td>“Parkinson’s—potentially amenable. AD-creates an issue because of memory problems. <strong>MS-depends on what format—individual consideration depending on condition. May be and may not be</strong>” – Decision Maker</td>
</tr>
</tbody>
</table>

### Q9. Why did you say <disease areas name> was <suitable/unsuitable>?

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Disease Area Focus: Cardiovascular

Negative points related to the existence of efficient measures and treatments, the dependence on disease severity and limitations due to nature of the disease

**Positive points** about using PPRNs to conduct effectiveness research for **cardiovascular conditions**

- Need more RWD in area
- Active, engaged patients
- Share information otherwise not collected
- Common diseases
- Self-reported symptoms

**Negative points** about using PPRNs to conduct effectiveness research for **cardiovascular conditions**

- Efficient measures or treatment already exist
- Depends on disease or severity
- Nature of disease may mean self-report unreliable

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# Reasons Cardiovascular Conditions Are Suitable for ER Using PPRNs

## Key responses

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<thead>
<tr>
<th>Response category</th>
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<tbody>
<tr>
<td>Common disease (n=4)</td>
<td>“Huge sample, you’re bound to get enough statistical power, enough range of severity, large population pool to pull from. It’s harsh but it’s true” – KOL</td>
</tr>
<tr>
<td>Active, engaged patients (n=5)</td>
<td>“Especially relatively young people who suffer from ACF could benefit, because probably never taken medication all their life and all of a sudden need to take multiple medications every day, <strong>may need a lot of support</strong> and <strong>may be very interested</strong> in finding out about their condition, drugs they are taking, what’s on the market, what options they have” – Decision Maker</td>
</tr>
<tr>
<td>Identify larger/subpopulations (n=1)</td>
<td>“Perhaps, that’s one where we have a lot of information, however, <strong>that information tends to be about one specific population</strong>, so <strong>broadening the reach</strong> in terms of the populations that can be evaluated, I do think there is an advantage” – KOL</td>
</tr>
</tbody>
</table>

Q9. Why did you say cardiovascular conditions (e.g. arrhythmia, chronic heart disease) were <suitable/unsuitable>?
## Reason Cardiovascular Conditions Are Unsuitable for ER Using PPRNs

### Key responses

<table>
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<tr>
<th>Response category</th>
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</thead>
<tbody>
<tr>
<td>Efficient measures or treatment (n=12)</td>
<td>“Not that important as <strong>have objective measurements [already].</strong> Symptoms are important but they correlate well with objective measures” — Decision Maker</td>
</tr>
<tr>
<td></td>
<td>“Don’t see the usefulness as it’s the <strong>least suitable</strong> field for me, as we <strong>have really good drugs</strong> and <strong>can treat with efficiency</strong>, so what can we do with this platform and this information?” — KOL</td>
</tr>
<tr>
<td></td>
<td>“<strong>Arrhythmia</strong> – got very objective measures of how it’s treated such as ECG – <strong>can have it without symptoms, so relying on subjective patients self-reporting</strong> on a condition which might not give you symptoms, I would question its usefulness” — Decision Maker</td>
</tr>
<tr>
<td>Nature of disease may mean self-report unreliable (n=5)</td>
<td>“Patient reported stuff is important, but there are some <strong>really good important markers of the disease</strong>, and with heart failure, <strong>need RCTs</strong> to see if drug A leads to less heart failure and death vs drug B” — Decision Maker</td>
</tr>
<tr>
<td>Depends on disease or severity (n=7)</td>
<td>“Think it will be reasonably useful there, although for <strong>arrhythmia</strong>, will be <strong>reliant upon electronic surveillance</strong>, monitors and the like, so there may be less utility there, but for things like <strong>congestive heart failure would be fairly useful</strong>” — KOL</td>
</tr>
</tbody>
</table>

Q9. Why did you say cardiovascular conditions (e.g. arrhythmia, chronic heart disease) were <suitable/unsuitable>?
Disease Area Focus: Cancer
The most frequently cited positive point is that it is important to know whether the patient thinks the treatment works.

Positive points about using PPRNs to conduct effectiveness research for cancer

- Important whether patient thinks treatment works
- Impact on lifestyle
- Frequent new medicines
- Share information otherwise not collected
- Self-reported symptoms
- Side effects/adverse events

Q9. Why did you say cardiovascular conditions (e.g. arrhythmia, chronic heart disease) were <suitable/unsuitable>?
## Reasons Cancer Is Suitable for ER Using PPRNs

### Key responses

<table>
<thead>
<tr>
<th>Response category</th>
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</tr>
</thead>
<tbody>
<tr>
<td>Impact on lifestyle (n=4)</td>
<td>“As treatments progress, outcomes and QOL become more and more focused on new treatments, <strong>real key area</strong> – oncology area, <strong>where PPRNs can really come into their own</strong>” – KOL</td>
</tr>
<tr>
<td>Important whether patient thinks treatment works (n=12)</td>
<td>“Patients are very engaged and want to be involved in treatment, also <strong>cancer drugs are fast tracked</strong> quite quickly, <strong>so getting that info from cancer patients</strong> might be quite <strong>useful</strong>” – Decision Maker</td>
</tr>
<tr>
<td>Expensive treatment (n=2)</td>
<td>“Cancer is <strong>the most important</strong> as we have a <strong>lot of info we don’t have from doctors</strong> that we can get from the patients, this is what we do every day, so of course extremely suitable. The <strong>weight of the diagnosis of cancer is not the same as the other diseases</strong>; the impact of cancer on a human is not the same, <strong>the financial impact, social impact</strong> is not the same, completely distinct” – KOL</td>
</tr>
</tbody>
</table>

Q9. Why did you say cancer was <suitable/unsuitable>?
Reasons Cancer Is Unsuitable for ER Using PPRNs

Key responses

<table>
<thead>
<tr>
<th>Response category</th>
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</table>
| Depends on disease area or severity n=6 | “Cancer itself I think there are some metrics the patient could bring in, but **its more to do with responsiveness to treatment and the side effects of the treatment**, but of course the question is once you’ve had cancer, are you cured? The **palliative aspects** if there really isn’t a cure, **becomes very amenable to patient (around pain, etc)**. So specifics of the questions, **types and stage** of cancer treatment **will decide the role of the patient**” – Decision Maker  

“Depending on where you’re at – **pain associated** with cancer, potentially **could benefit if talking of pain control**, but if developing **chemotherapy to reduce tumour then no**” – Decision Maker |

* Chart not created for cancer and unsuitable rationales as there were too few respondents in this category

Q9. Why did you say cancer was <suitable/unsuitable>?

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Caveats and Limitations
Caveats and Limitations

• One limitation is that all of the decision makers were based in the UK. This may mean that they were less likely to have heard of PPRNs than decision makers in the US, which could have influenced their responses.

• Additionally, most KOLs were from the US which could have an influence upon this groups responses.

• KOLs often had a special interest in PPRNs or had conducted research into them. While this meant that they could provide special insight into PPRNs it also meant that they may have had a particularly favourable view of them.
Findings and Implications
Evaluation of PPRN Definition Provided

Findings

- The majority of KOLs were positive about the PPRN definition provided (see slide 17 for definition)
- A few KOLs who were unsure about the definition said this related to PPRNs not necessarily having to be online

Implication

- While future development should be expected the current PPRN definition is mostly acceptable
Usefulness of PPRNs for Conducting Effectiveness Research

<table>
<thead>
<tr>
<th>Findings</th>
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<tbody>
<tr>
<td>Although most KOLS and healthcare decision makers reported that they thought PPRNs could be useful for conducting effectiveness research, when asked why many reported limitations alongside positive PPRN attributes. This implies that even when generally positive about PPRNs most respondents also have limitations/barriers to using them top of mind.</td>
</tr>
<tr>
<td>Commonly cited advantages of PPRN cited by both respondent types were the inclusion of the patients’ perspective and their involvement as well as the addition of hitherto inaccessible information (such as that relating to patient QOL).</td>
</tr>
<tr>
<td>When thinking of the limitations of PPRNs both KOLs and healthcare decision makers often mention biases (in both self reporting and PPRN participant populations composition).</td>
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<table>
<thead>
<tr>
<th>Implication</th>
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<tbody>
<tr>
<td>Although positive views of PPRNs abound, the caveats that relate to their use in CER are top of mind and would need to be addressed as part of a strategy to convince stakeholders of their validity.</td>
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Acceptability of Using PPRNs for Effectiveness Research

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<tr>
<th>Findings</th>
<th>Implication</th>
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<tbody>
<tr>
<td>More than 2/3 of healthcare decision makers felt that using PPRN based</td>
<td>The willingness for using PPRN based effectiveness research is present, the</td>
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<tr>
<td>effectiveness research for making healthcare decisions in their</td>
<td>barrier (as seen in the associated pragmatic literature review &amp; desktop</td>
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<tr>
<td>professional role is acceptable</td>
<td>research) is that the majority of PPRNs may not yet be in a position to</td>
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<tr>
<td></td>
<td>provide it</td>
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<tr>
<td>• The need for taking the patients view into account was among the</td>
<td>•</td>
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<tr>
<td>top reasons driving this</td>
<td>• The willingness for using PPRN based effectiveness research is present,</td>
</tr>
<tr>
<td>• Among the 1/3 who did not say it was acceptable a lack of knowledge</td>
<td>the barrier (as seen in the associated pragmatic literature review &amp;</td>
</tr>
<tr>
<td>in relation to PPRNs/RWE was mentioned among leading factors.</td>
<td>desktop research) is that the majority of PPRNs may not yet be in a</td>
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<td>position to provide it</td>
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The Role of PPRNs in Effectiveness Research in Europe in 5-10 years

Findings

- Just over 2/3 of KOLs felt PPRNs would have a role in future research in Europe. Top mentioned reasons for this included the need for patient reported outcomes/RWE in research as well as the idea that Europe is already involving patients in decision making.

- Among KOLs who were unsure a top mentioned barrier was that the regulators were not using patient reported outcomes.

Implication

- The mentioned KOL cited barrier (that regulators are not using patient reported outcomes) should be viewed in contrast to answers given in this survey by healthcare decision makers (category includes regulators) which implied that they see value in having access to the patients views – it could simply be that healthcare decision makers need to be made aware/given access to such information.
## Impact of PPRNs on Patient Outcomes

<table>
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<th>Implication</th>
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<tbody>
<tr>
<td>- At an individual patient level respondents often cite increased engagement in healthcare, improved health outcomes and the inclusion of the patients voice in research</td>
<td>• Respondents have suggested numerous positive impacts of PPRNs on patient outcomes in this survey. GetReal could produce specific case studies that demonstrate such suggested impacts and this would be a great way to highlight the value of these networks to both patients (thereby increasing participation) and healthcare decision makers (thereby increasing their positive view of PPRNs)</td>
</tr>
<tr>
<td>- At a population level while many respondents said it is hard/too soon to know, some said it would help in identifying the best treatments for disease populations</td>
<td></td>
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</table>
Evaluation of PPRN Types (Patient Reported Outcomes vs EMR Based)

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<thead>
<tr>
<th>Findings</th>
<th>Implication</th>
</tr>
</thead>
<tbody>
<tr>
<td>PPRNs containing both patient reported outcomes and EMRs were seen as far superior to those containing just one of the two (by both KOLs and healthcare decision makers)</td>
<td>PPRNs containing both patient reported outcomes and EMRs are seen as the best option by both KOLs and healthcare decision makers, however, as we saw in our desktop research (separately published) these are in the minority</td>
</tr>
<tr>
<td>EMR only PPRNs were seen positively for being factual/accurate but negatively in that they lacked the richness or patient reported outcome based PPRNs. Another criticism was that physicians may provide inaccurate reports</td>
<td>As EMRs are the harder to access data source, an approach which involves support from healthcare decision makers would be needed in order to increase the number of functioning EMR/patient reported outcome PPRNs</td>
</tr>
<tr>
<td>Patient reported outcome only PPRNs are seen positively in relation to the provision of unique data but criticised for the inconsistent nature of self reporting, the lack of essential data which is present in EMR and the difficulty in validating the data</td>
<td></td>
</tr>
<tr>
<td>Many of the above mentioned concerns are addressed with PPRNs containing both EMRs and patient reported outcomes with respondents saying that they allow for validation of content and provide both the physicians and the patients views</td>
<td></td>
</tr>
</tbody>
</table>

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Obstacles and Potential Solutions for Making PPRNs More Useful for Effectiveness Research

Note: only KOLs were asked to give input on this

<table>
<thead>
<tr>
<th>Obstacle</th>
<th>Suggested solution of interest</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ethical and regulatory obstacles</td>
<td><strong>Test what you do in a medical setting which will give you feasibility and credibility with your patients and doctors</strong></td>
</tr>
<tr>
<td></td>
<td><strong>Educate people and show benefit of sharing information and why it’s useful</strong></td>
</tr>
<tr>
<td>Data collection and self-report validation obstacle</td>
<td><strong>Partner with other organisations with databases and run comparison studies with them...publish work on differences and how to understand implications</strong></td>
</tr>
<tr>
<td></td>
<td><strong>Come up with standard agreed definitions of what you mean by terms in the same way we did in this interview</strong></td>
</tr>
<tr>
<td>Quality of PPRN data</td>
<td><strong>Make it clear what they can and cannot do</strong></td>
</tr>
</tbody>
</table>
Usefulness of PPRNs for Conducting Effectiveness Research by Disease Area

**KOLs:** while the vast majority of KOLs viewed all disease areas as being suitable, a small proportion were either unsure or negative about the following areas: mental and behavioural, neurological and cardiovascular.

**Healthcare decision makers:** while a high proportion of decision makers deemed 4 out of the 7 tested disease areas to be suitable, a high proportion of them were also sceptical about mental and behavioural, neurological and cardiovascular.

Common **positive points** about using PPRNs in relation to a disease area include:

- Where the importance of whether the patient thinks the treatment works is high (ex. in cancer where there are often fast tracked medicines)
- Where patient self reports provide information unavailable through clinical measures (ex. with respiratory conditions where patients report on tolerance of exercise, ability to work is needed alongside laboratory tests like CO2 blood gas level tests)

Frequently mentioned **negative points** in relation to using PPRNs for a disease area:

- Where the disease area metrics are already very effective (ex. within the cardiovascular category and for arrhythmia current clinical measures which are deemed superior to patient self reporting)
- In relation to neurological and mental health areas, the nature of the disease is cited as meaning that in more severe cases the patients self reports may be unreliable

**Implication:** while KOLs are positive about the use of PPRNs for CER across all disease areas, healthcare decision makers are not for some diseases. This implies that either decision makers need to be informed as to why certain disease areas are suitable (despite their misgivings) or for now certain disease areas could be deprioritised by PCORI.
Comparison against Results from the Pragmatic Literature Review & Desktop Research
Results from the survey:
- The importance of PPRNs containing EMRs and PROs is highlighted with both KOLs and healthcare decision makers giving substantially higher ratings to these PPRNs vs others.

Results from the desktop research:
- Only some (1/3) PPRNs had the capacity to collect both EMR and PRO data (while these PPRNs have the capacity to collect both types of data, it is uncertain to what extent they contain both types of data).

**Conclusion:** Stakeholders (KOLs and healthcare decision makers) see PPRNs containing EMRs and PROs as the optimum solution, however, these exist in relatively low abundance.
Overall Take Away – Insights From Comparison of Desktop Research/Literature Review With Survey Results (2/2)

Advancements may be required for PPRNs to be used in CER, however, healthcare decision makers were positive about their potential uses outside of CER and in relation to providing the patients subjective view of treatment.

- Some **key advancements need to be made** in order to realise the true potential of PPRNs:
  - More PPRNs containing both PRO (self-reported) and EMR data
  - Standardisation of data (EMR and self-reported)
  - Validation of self-reported data
  - More consistent collection of parameters necessary for CER e.g. treatment information
  - Concerns relating to privacy and consent allayed

- In spite of this there is a **high degree of receptiveness** among decision makers in terms of what PPRNs can deliver especially in relation to capturing the **patients view and subjective experience of treatment** (something that PPRNs currently can be said to do even where the data might not yet be acceptable for CER).

- **Conclusion:** Therefore in the **short term**, PPRNs could be used by healthcare decision makers to understand the **patient point of view** while in the **longer term**, once advancements are made, they could also be used for CER.
Appendix - Usefulness of PPRNs by Disease Area (follow on from slide 59)
Disease Area Focus: Respiratory Conditions

KOLs provided more positive points about using PPRNs to conduct ER for respiratory conditions than decision makers.

**Positive points about using PPRNs to conduct ER for respiratory conditions**

- Chronic conditions
- Important whether patient thinks treatment works
- Existing research in area
- Need more RWD in area
- Common diseases/large cohort
- Self-reported symptoms
- Self-managed condition

Q7. Respiratory conditions (e.g. asthma, COPD)?
# Reasons Why Respiratory Conditions Are Suitable for ER

## Key responses

<table>
<thead>
<tr>
<th>Response category</th>
<th>Response</th>
</tr>
</thead>
<tbody>
<tr>
<td>Need for more RWD (n=5)</td>
<td>“Ideal. <em>We actually have conducted effectiveness research</em> now in that area. We are now trying to do it in real world setting” – Decision Maker</td>
</tr>
<tr>
<td>Important what patient thinks is working (n=6)</td>
<td>“<em>Patient experience</em> of asthma <em>adds a lot to laboratory tests</em> e.g. CO2 level, actually want to know patient experience and tolerance of exercise, ability to work, <em>which don’t get through clinical measures</em>” – Decision Maker</td>
</tr>
<tr>
<td>Chronic condition (n=5)</td>
<td>“I think it would be useful for these patients and how they manage long term conditions. <em>They will live with it for the rest of their life</em> and so I think it could be useful” – Decision Maker</td>
</tr>
<tr>
<td>Self managed condition (n=4)</td>
<td>“Chronic condition – patient used to managing it on their own – <em>large gaps between clinician visits, so capturing in between times</em>” – KOL</td>
</tr>
</tbody>
</table>

Q9. Why did you say <disease areas name> was <suitable/unsuitable>?
Reasons Why Respiratory Conditions Are Unsuitable for ER

Key responses

<table>
<thead>
<tr>
<th>Response category</th>
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</tr>
</thead>
<tbody>
<tr>
<td>Depends on disease area (n=3)</td>
<td>“Asthma more [suitable] than COPD, COPD caused by cigarette smoking so if people haven’t heeded advice to stop smoking, then likely don’t have good knowledge. Asthma is genetic, can’t prejudge” – Decision Maker</td>
</tr>
<tr>
<td>Nature of disease may mean less likely to use PPRNs (n=1)</td>
<td>“Experience is that it’s difficult to engage people with asthma and COPD – COPD patients are older, may have lifestyle factors leading to COPD which mean they are less likely to participate in PPRN. Asthma – mild, learnt to live with it by this time, don’t seek extra help such as PPRNs” – KOL</td>
</tr>
<tr>
<td>Difficult to engage due to age (n=1)</td>
<td>“Asthma – yes, COPD - no. Different demographics of patient populations, with EMR would be fine for both, but for self reported, COPD patients are older, more comorbidities, don’t use smart phones and computers, not the same response as younger technology savvy population” – KOL</td>
</tr>
</tbody>
</table>

* Chart not created for respiratory and unsuitable rationales as there were too few respondents in this category

Q9. Why did you say <disease areas name> was <suitable/unsuitable>??
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### Reasons Why Gastrointestinal Conditions Are Suitable for ER

#### Key responses

<table>
<thead>
<tr>
<th>Response category</th>
<th>Response</th>
</tr>
</thead>
<tbody>
<tr>
<td>Impact on lifestyle (n=6)</td>
<td>&quot;They’re things where patients are well positioned to discuss impact on everyday life and overall wellbeing, but not so sick that they’re not able to report&quot; – KOL</td>
</tr>
<tr>
<td>Chronic condition (n=5)</td>
<td>“Chronic condition – patient used to managing on their own – large gaps with clinician, so capturing in between times” – KOL</td>
</tr>
<tr>
<td>Expensive treatments (n=4)</td>
<td>“More severe ones [diseases], where we are spending a lot of money. We could potentially look at patient adherence” – Decision Maker</td>
</tr>
<tr>
<td>Can report at any time (n=4)</td>
<td>“It could be useful because they can report what there stools are like which can be indication of GI problems, can report easily – can be measured in realistic way” – Decision Maker</td>
</tr>
</tbody>
</table>

Q9. Why did you say <disease areas name> was <suitable/unsuitable>?
Reasons Gastrointestinal Conditions Are Unsuitable for ER

Key responses

<table>
<thead>
<tr>
<th>Response category</th>
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</tr>
</thead>
<tbody>
<tr>
<td>Depends on disease or severity (n=3)</td>
<td>“Useful, depends on which GI condition; something specialised need hospital might be more difficult than primary care” – Decision Maker</td>
</tr>
<tr>
<td>Depends what patient reports on (n=2)</td>
<td>“Depends what asking them to report, if it’s symptoms after trying something new, then maybe, but it’s quite closely managed in terms of seeing healthcare professional quite a lot, and mainly managed by secondary care, so unsure” – Decision Maker</td>
</tr>
</tbody>
</table>

* Chart not created for GI and unsuitable rationales as there were too few respondents in this category

Q9. Why did you say <disease areas name> was <suitable/unsuitable>?
Disease Area Focus: Rheumatological

Reasons it may be suitable

Positive points about using PPRNs to conduct effectiveness research for rheumatological conditions

- Self-reported symptoms
- Need more RWD in area
- Important whether patient thinks treatment works
- Chronic conditions
- Active, engaged patients
- Can report at any time
- Variable disease - ups and downs

KOLs vs. Healthcare Decision Makers

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## Key responses

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</tr>
</thead>
<tbody>
<tr>
<td>Self reported symptoms (n=8)</td>
<td>“Can be painful, people tend to know what to do under certain circumstances, because of need of ongoing treatment. So could be effectively used as part of research” – Decision Maker</td>
</tr>
<tr>
<td>Variable disease-ups and downs (n=3)</td>
<td>“They have flares, particularly with rheumatoid, people are on multiple medications, side effects issues, do people comply? Yes I think so. I think it could be helpful” – KOL</td>
</tr>
</tbody>
</table>

Q9. Why did you say <disease areas name> was <suitable/unsuitable>?
# Reasons Rheumatological Conditions Are Unsuitable for ER

## Key responses

<table>
<thead>
<tr>
<th>Response category</th>
<th>Response</th>
</tr>
</thead>
<tbody>
<tr>
<td>Nature of disease may mean patients less likely to use PPRNs (n=1)</td>
<td>“You will have to have access to a computer and mobility so people with arthritis who have less mobility, it will be more difficult for them to access and use a computer” – Decision Maker</td>
</tr>
<tr>
<td>Nature of disease may mean self reported unreliable (n=2)</td>
<td>“...there are more disease markers that are biochemical, so when doing studies of these drugs, as well as working out if patient feels better, you actually do look at their biochemical markers, so would put that in the middle really” – Decision Maker</td>
</tr>
</tbody>
</table>

* Chart not created for rheumatologic conditions and unsuitable rationales as there were too few respondents in this category

Q9. Why did you say <disease areas name> was <suitable/unsuitable>?
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Reasons Why Mental and Behavioural Health Conditions May Be Suitable for ER

Key responses

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<thead>
<tr>
<th>Response category</th>
<th>Response</th>
</tr>
</thead>
<tbody>
<tr>
<td>Self reported symptoms (n=8)</td>
<td>“More important – even diagnostic criteria for mental health diseases depends on patient and provider interaction, more in the subjective or patient reported realm, so having patient part of the research enterprise for that disease seems essential” – KOL</td>
</tr>
<tr>
<td>Hard to do in RCTs (n=1)</td>
<td>“Definitely, this is one of the key areas where RWD can really help. Lots of literature on how clinical trials for mental health are poorly conducted in the past and there really needs to be a good look at how we assess the effectiveness of interventions for mental health” – Decision Maker</td>
</tr>
</tbody>
</table>

Q9. Why did you say <disease areas name> was <suitable/unsuitable>?
Negative points about using PPRNs to conduct ER for mental and behavioural health conditions

- Nature of disease may mean self-report unreliable
- Depends on disease or severity
- Not familiar with domain
- Nature of disease may mean patients less likely to use PPRN
- Diagnosis definition discrepancies
- Difficult to get objective outcomes measures
- Need to monitor patients safety
- Need carer/objective observer to report
- In combination with other data

KOLs vs Healthcare Decision Makers

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# Reasons Why Mental and Behavioural Health Conditions May Be Unsuitable for ER

## Key responses

<table>
<thead>
<tr>
<th>Response category</th>
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</tr>
</thead>
</table>
| Nature of Disease may mean Self report unreliable (n=13) | “Depends upon how ill patient is and what their condition is, with mental health, perception of symptoms may be clouded as mentally unwell, might have to take responses with pinch of salt, as may give different responses when recovered” – Decision Maker  
“More difficult because using EMR data is already difficult now. Collecting information from mentally diseased patients is a challenge. It could be good for other reasons, like looking for unmet needs, but you need to be very careful” – KOL |
| Depends on disease severity (n=8) | “Depression and ADHD could be, but other mental disorders trickier, not saying it couldn’t be used, but with severe mental disorders, self-reported information becomes a little more suspect e.g. schizophrenia” – KOL |
| Not familiar with domain (n=5) | “I think the EMR may not be as complete because it is so hard for a clinician to get this information from patient. At least in the US, we often don’t put things down in a record because we don’t want to jeopardise patient in future or label them. Careful about saying someone is an alcoholic because we don’t want the label to harm them down the road. Providers know that there are things not in EMR for that reason” – KOL |

Q9. Why did you say <disease areas name> was <suitable/unsuitable>?