PATIENT POWERED REGISTRIES: USEFUL FOR HEALTH TECHNOLOGY ASSESSMENT OR NOT?

Gurmit Sandhu, Elisabeth M. Oehrlein, Robert N. McBurney & Chantal Guilhaume

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PATIENT POWERED REGISTRIES PPRs: USEFUL FOR HEALTH TECHNOLOGY ASSESSMENT HTA OR NOT?

Discussion Leaders

Gurmit Sandhu, B Pharm (Hons), MPH, MBM
Patient Engagement Specialist, Gurmit Sandhu Consulting GmbH, Basel, Switzerland

Elisabeth M. Oehrlein, PhD Candidate
at University of Maryland, Baltimore, USA and Co-Chair ISPOR Special Interest Group on Digest of Databases

Robert N. McBurney, PhD,
Co-Principal Investigator, iConquerMS™ - the MS Patient-Powered Research Network

Chantal Guilhaume, PharmD,
Haute Autorité de Santé, France, scientific Project Manager, EUnetHTA JA3, Direction de l’Evaluation Médicale, Economique et de Santé Publique (DEMESP)

Workshop Overview

- Overview of registries
- MS case study, HTAs

Elisabeth

Robert

- MS Patient-Powered Research Network

Chantal

- Patient Voice, Eunetha JA 3
- Requirements for registries

Audience Poll
Changes in Narrative: Patient Voice in HTA on “Added Value” Evidence and Description

Assessment Process  Evidence for Value  Future??

Relevant Patient Reported Outcomes, Patient subgroups & Others

Patient Relevant Outcomes

<table>
<thead>
<tr>
<th>Aspects</th>
<th>Examples</th>
</tr>
</thead>
</table>
| Practical   | • Sporting activities  
              • Education/training  
              • Social opportunities  
              • Relationship & intimacy |
| Physical    | • Activities of daily life  
              • Work & income  
              • Fatigue, pain  
              • Lack of restful sleep |
| Social      | • Narrowing of social roles  
              • Feeling excluded & isolated |
| Emotional   | • Feeling frustrated  
              • Misunderstood  
              • Depressive & low |
| Others      | • Adverse events  
              • Adherence & concordance |

A framework on “Added Value” Evidence & Patient Powered Registries

Generation

Interpretation

Application

governance, quality, scope, equality, feasibility etc

clinical outcomes link to relevant patient reported outcomes etc

Timeliness, in health care delivery and research and/or market/patient access? etc

Introduction to Patient Powered Registries and Multiple Sclerosis Case Study

Elisabeth M. Oehrlein

November 6, 2017
Presentation Overview

• Patient (powered) registries
  – Types
  – Data sources
  – Patient-powered
• Case study on MS

**Patient registry**: a collection—for one or more purposes—of standardized information about a group of patients who share a condition or experience.
Data sources

- Professional organizations
- Manufacturers
- Government
- Geographic region
- Independent hospital
- Integrated delivery systems
- Patient advocacy organizations

<table>
<thead>
<tr>
<th>Data source</th>
<th>Electronic medical records</th>
<th>Administrative claims</th>
</tr>
</thead>
<tbody>
<tr>
<td>Demographics characteristics</td>
<td></td>
<td>Patient-reported outcome measures</td>
</tr>
<tr>
<td>Biospecimens</td>
<td></td>
<td>Quality of life measures</td>
</tr>
<tr>
<td>Satisfaction with care</td>
<td></td>
<td>Lab results</td>
</tr>
<tr>
<td>Wearable technologies</td>
<td></td>
<td>Other emerging data sources</td>
</tr>
</tbody>
</table>

Patient-Powered Registry (or network)

Researchers

Patients / patient organization(s)

Created
Maintained
Controlled

Advisory role

Advantages of Patient-Powered Registries (or networks)

Patient-centered data on...
- Disease burden
- Patient journey
- Unmet medical need
- Patient preferences
- Natural history of disease
- Subgroups
- Outcomes and endpoints

Opportunities

Patient-centered data on...
- Disease burden
- Patient journey
- Unmet medical need
- Patient preferences
- Natural history of disease
- Subgroups
- Outcomes and endpoints

Challenges
- Acceptability
- Precedence
- Standardization
- Quality
- Validity
- Heterogeneity


Patient engagement activities to develop recommendations

<table>
<thead>
<tr>
<th></th>
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<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Disease / treatment</td>
<td>• Patient / patient advocate interviews</td>
<td>• Patient group responded to common drug review call for patient input</td>
<td>Two patient experts:</td>
</tr>
<tr>
<td>burden</td>
<td>• Survey facilitated by the MS Coalition (nearly 16,000 participants)</td>
<td></td>
<td>• First two Committee discussions</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>• Submitted written evidence</td>
</tr>
<tr>
<td>Patient preferences</td>
<td>Some patients prefer oral delivery, others equally comfortable with injections</td>
<td>Oral delivery preferred over injections</td>
<td></td>
</tr>
<tr>
<td>Outcomes and endpoints</td>
<td>Long-term outcomes</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Identifying PPRs

• ISPOR Digest of Databases is being updated and will include patient registries

• Agency for Healthcare Research and Quality’s Registry of Patient Registries
  [https://patientregistry.ahrq.gov/](https://patientregistry.ahrq.gov/)

• Patient registries in Europe
iConquerMS™: People-Powered Research Network bridged to Researchers and Other Stakeholders

- More than 4,200 participants, growing daily
- Funded by PCORI as part of PCORnet
- Governed by majority of PwMS - the experts
- Research portfolio developing rapidly

**OPEN SCIENCE driven by People with MS**

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**iConquerMS™ Participants Contribute Data Frequently**

<table>
<thead>
<tr>
<th>Survey</th>
<th>REAL MS™ Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>Demographics</td>
<td>Initial, Summer 2016, Winter 2017, Summer 2017</td>
</tr>
<tr>
<td>MS Characteristics</td>
<td>Initial, Summer 2016, Winter 2017, Summer 2017</td>
</tr>
<tr>
<td>Neuro-QoL Adult Short Form</td>
<td>Initial, Summer 2016, Winter 2017, Summer 2017</td>
</tr>
<tr>
<td>(now called “Quality of Life”)</td>
<td></td>
</tr>
<tr>
<td>PROMIS® Global Health</td>
<td>Initial, Summer 2016, Winter 2017, Summer 2017</td>
</tr>
<tr>
<td>(now called “Overall Health”)</td>
<td></td>
</tr>
<tr>
<td>Physical Activity</td>
<td>Summer 2016, Winter 2017, Summer 2017</td>
</tr>
<tr>
<td>Other (Medical) Conditions</td>
<td>Summer 2016, Winter 2017, Summer 2017</td>
</tr>
<tr>
<td>Use of Complementary &amp;</td>
<td></td>
</tr>
<tr>
<td>Alternative Medicine</td>
<td>Summer 2017</td>
</tr>
</tbody>
</table>

✪ added Bowel, Bladder and Vision from MSQLI

REAL MS™: Research Engagement About Life with MS
### What Affects iConquerMS™ Participants Most?

<table>
<thead>
<tr>
<th>Rank Order</th>
<th>Neuro-QoL Domain (5-point Likert scale questions)</th>
<th>Average Score (N = ~1,400)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Fatigue</td>
<td>2.89</td>
</tr>
<tr>
<td>2</td>
<td>Satisfaction with Social Roles and Activities</td>
<td>3.09</td>
</tr>
<tr>
<td>3</td>
<td>Sleep Disturbance</td>
<td>3.59</td>
</tr>
<tr>
<td>4</td>
<td>Positive Affect and Well Being</td>
<td>3.59</td>
</tr>
<tr>
<td>5</td>
<td>Ability to Participate in Social Roles and Activities</td>
<td>3.60</td>
</tr>
<tr>
<td>6</td>
<td>Anxiety</td>
<td>3.67</td>
</tr>
<tr>
<td>7</td>
<td>Cognitive Function</td>
<td>3.71</td>
</tr>
<tr>
<td>8</td>
<td>Emotional and Behavioral Dyscontrol</td>
<td>3.92</td>
</tr>
<tr>
<td>9</td>
<td>Lower Extremity Functional Mobility</td>
<td>3.93</td>
</tr>
<tr>
<td>10</td>
<td>Depression</td>
<td>4.11</td>
</tr>
<tr>
<td>11</td>
<td>Stigma</td>
<td>4.17</td>
</tr>
<tr>
<td>12</td>
<td>Communication</td>
<td>4.41</td>
</tr>
<tr>
<td>13</td>
<td>Upper Extremity Function Fine Motor ADL</td>
<td>4.54</td>
</tr>
</tbody>
</table>
What Affects iConquerMS™ Participants Most? - More Detail

Details are Important

Low scores are worse

Background - What Matters Most to PwMS

Survey of iConquerMS™ participants conducted in January 2017
826 respondents
Patient Voice EUnetHTA JA3
Requirements for registries

Chantal Guilhame,
Scientific Project Manager, EUnetHTA JA3
Direction de l’Évaluation Médicale, Economique et de Santé Publique /Haute Autorité de Santé (HAS) - France
ISPOR, Glasgow 2017

EunetHTA JA3 organisation

81 partners consisting of national, regional and non-for-profit agencies that produce or contribute to HTA
Patient Voice all along the health technology life-cycle

**Time line of innovation**

**WP5**
- **Early Dialogues**
- **Scientific Advice**
- **Assessment for market authorization**
- **Additional data collection**

**WP4**
- **Rapid REA**

**WP5**
- **Comparative or full HTA / REA**

**Use of technology in health care**

**Collecting evidence in early development.**

**Collecting post-marketing evidence.**

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**Patient Voice in evidence generation**

**Work Package 5: Life cycle approach to improve evidence generation**

**WP5- Strand A: Early Dialogues (ED)**

- **Lead:** HAS and **co-Lead:** G-BA

- Support developers of medical technologies on their product development plan by providing a collaborative approach between a wide range of European HTA agencies:
  - Opportunity to recommend specific Patient Reported Outcomes
  - Opportunity to discuss development of Real World Evidence

- One process for parallel regulator-HTA Early Dialogues/Scientific advice since July

- Involvement of patients in the EDs during JA3 will be built on experience from JA2 and SEED, but also on EMA and other national experiences:
  - EUneHTA Transversal Task Force on patient and health care professional contribution
  - Test several ways to involve patients (interviews, F2F meeting participation…) before final procedure fitting national legal constraints.
Patient Voice in evidence generation

Work Package 5 : Life cycle approach to improve evidence generation

WP5-Strand B: Post-launch Evidence Generation (PLEG) and Registries

Lead: HAS

• Define process of generating post-launch evidence from clinical practice over the cycle of health technology and using it for re-assessment and reimbursement decisions

• Registries to be considered as one of the data source
  • Development of standard tool to assess registry quality
  • Collaboration with EMA on qualification advise of disease registry

Usage of Patient-based evidence by HTA bodies

1. As a supplement to clinical measures in Randomized Clinical Trials, Patient-based evidence support
   - Confirmation of efficacy and tolerability particularly useful in orphan indication
   - Interpretation of efficacy data, relevance from a patient’s perspective
   - Potential long-term outcomes in real life conditions
   - Collection of epidemiology data and natural disease evolution data

2. Inform on terms of use
   - Appropriate use of drug to secure optimal benefit (off-label usage)
   - Treatment algorithm in practice

3. Cost-utility evaluation
   - Need for generic utility scales
Challenges associated with real world data

1. Representativity
   - Various patients profiles
   - Country Specificity (various disease management)

2. Data Quality
   - Bias
   - Missing data

3. Descriptive vs comparative data
   - Difficulties related to interpretation of contradictory results

4. Independency
   - Request for transparency on source of funding to prevent potential conflict of interest

Workshop Discussion and ISPOR Audience Poll*

<table>
<thead>
<tr>
<th>Statement</th>
<th>Choose one of the following responses for each statement</th>
</tr>
</thead>
<tbody>
<tr>
<td>S1  “I understand what are Patient Powered Registries”</td>
<td>Yes  No  Not Sure</td>
</tr>
<tr>
<td>S2  “Patient Powered Registries offer benefits over traditional registries for HTA agencies”</td>
<td>Yes  No  Not Sure</td>
</tr>
<tr>
<td>S3  “Patient Powered Registries provide data that is complementary to existing patient engagement methods used in HTA (e.g., advisory panels)”</td>
<td>Yes  No  Not Sure</td>
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Poll: "I understand what are Patient Powered Registries"

Poll: “Patient Powered Registries offer benefits over traditional registries for HTA”
Poll: “Patient Powered Registries provide data that is complementary to existing patient engagement methods used in HTA (e.g., advisory panels)”

Questions

• Should all clinical trials in MS include PROs that provide data on symptoms, functioning and quality of life to complement clinical assessments such as relapse rates, EDSS and MRI features?

• Do we need an Operating System for generating Real World Evidence?

• Are “one off” panels, etc., to gain the “patient voice” the right method for gaining input from people with MS in regulatory approvals or HTA?
Summary: Patient Powered Registries & HTA.

- **Generation**
  - governance, quality, scope, equality, feasibility etc

- **Interpretation**
  - clinical outcomes link to relevant patient reported outcomes etc

- **Application**
  - Timeliness, in health care delivery and research and/or market/patient access? linguistics, etc

Continue the discussion on ISPOR app & .....