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

ISPOR Workshop
6th Nov 2017
11.15 – 12.15pm

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

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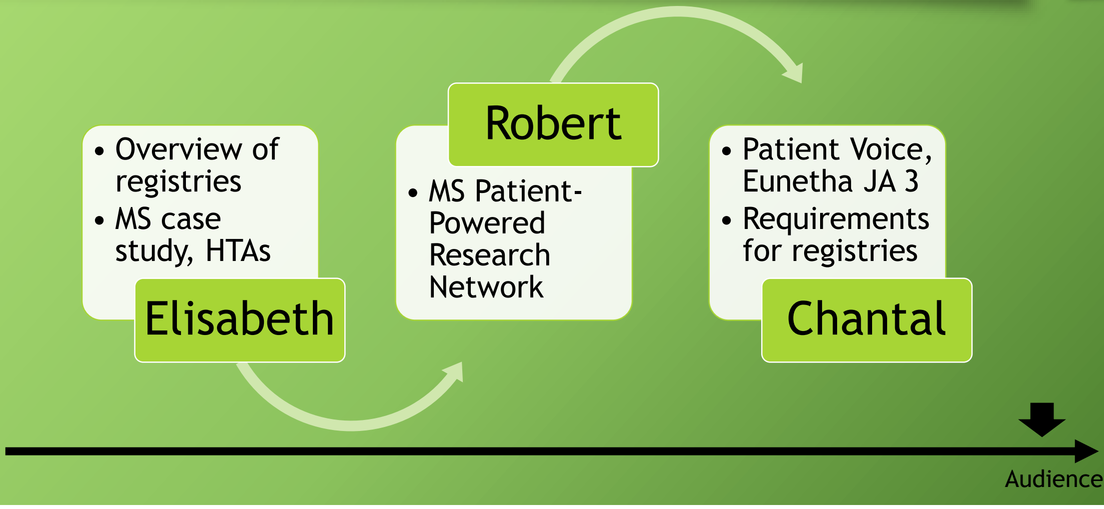
PATIENT POWERED REGISTRIES PPRs: USEFUL FOR HEALTH TECHNOLOGY ASSESSMENT HTA OR NOT?

ISPOR
Workshop,
Glasgow
Nov 2017

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 Autoritè de Santè, France, cientific Project Manager, EUnetHTA JA3, Direction de l'Evaluation Médicale, Economique et de Santé Publique (DEMESP)

Workshop Overview



Elisabeth
- Overview of registries
- MS case study, HTAs

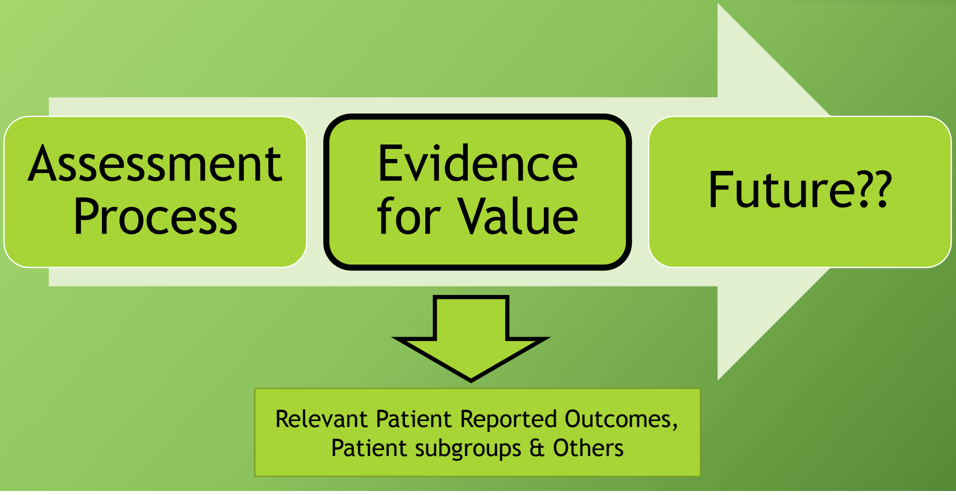
Robert
- MS Patient-Powered Research Network

Chantal
- Patient Voice, Eunetha JA 3
- Requirements for registries

Audience Poll

Changes in Narrative: Patient Voice in HTA on " Added Value" Evidence and Description

Societal movement to integrate patient perspectives


Assessment Process

Evidence for Value

Future??

Relevant Patient Reported Outcomes, Patient subgroups & Others

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Patient Relevant Outcomes

Aspects	Examples [1]
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

1. Guillevin L et al . Understanding the impact of pulmonary arterial hypertension on patients' and carers' lives. Eur Respir Rev 2013;22:535-8

A framework on "Added Value" Evidence & Patient Powered Registries

- **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? etc

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UNIVERSITY of MARYLAND

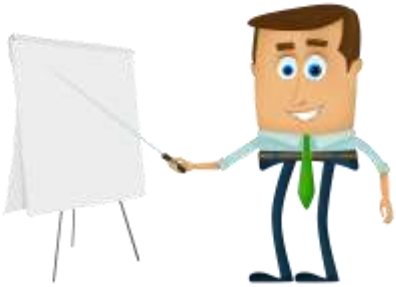
Introduction to Patient Powered Registries and Multiple Sclerosis Case Study

Elisabeth M. Oehrlein

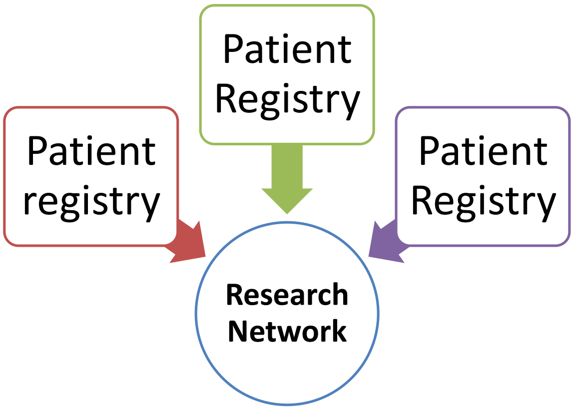
November 6, 2017

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



Gliklich RE, Dreyer NA, Leavy MB, editors. Registries for Evaluating Patient Outcomes: A User's Guide [Internet]. 3rd edition. Rockville (MD): Agency for Healthcare Research and Quality (US); 2014 Apr. 1, Patient Registries. from: https://www.ncbi.nlm.nih.gov/books/NBK208643/

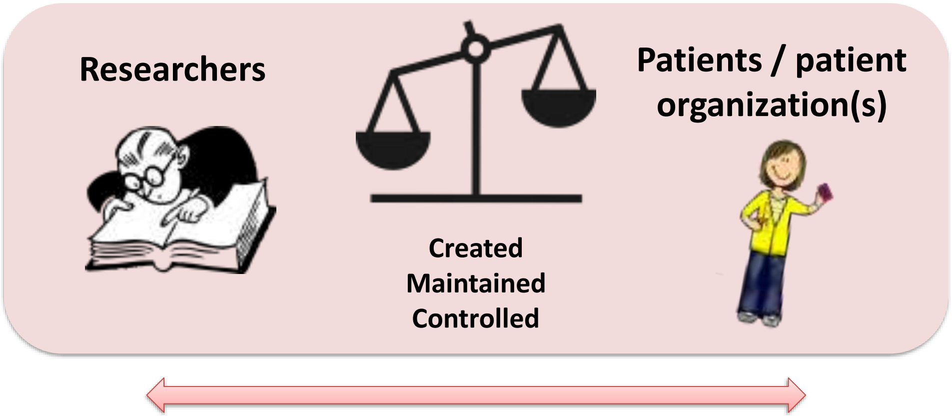
Gliklich RE. Patient Registries. Presented at Patient Centered Outcomes Research Institute (PCORI). https://www.pcori.org/assets/11-Gliklich-Slides-Registries.pdf

Data sources

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

Electronic medical records	Administrative claims
Demographics characteristics	Patient-reported outcome measures
Biospecimens	Quality of life measures
Satisfaction with care	Lab results
Wearable technologies	Other emerging data sources

Patient-**Powered** Registry (or network)



Researchers

Patients / patient organization(s)

Created
Maintained
Controlled

Advisory role

Workman TA. Engaging Patients in Information Sharing and Data Collection: The Role of Patient-Powered Registries and Research Networks [Internet]. Rockville (MD): Agency for Healthcare Research and Quality (US); 2013 Sep. Defining Patient Registries and Research Networks. https://www.ncbi.nlm.nih.gov/books/NBK164514/

Advantages of Patient-**Powered** Registries (or networks)

Patient-centered data on…

Disease burden

Patient journey

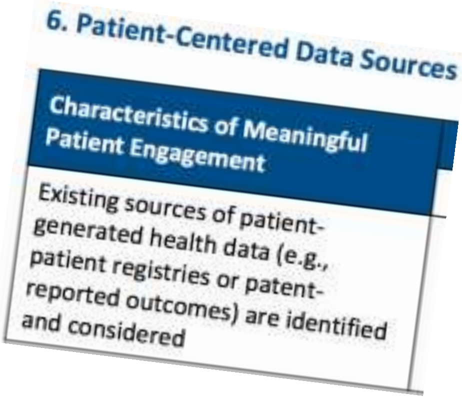
Unmet medical need

Patient preferences

Natural history of disease

Subgroups

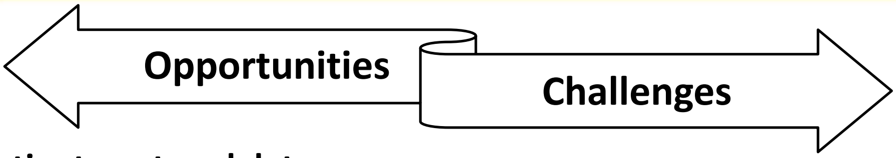
Outcomes and endpoints



6. Patient-Centered Data Sources

Characteristics of Meaningful Patient Engagement
Existing sources of patient-generated health data (e.g., patient registries or patent-reported outcomes) are identified and considered

Workman TA. Engaging Patients in Information Sharing and Data Collection: The Role of Patient-Powered Registries and Research Networks [Internet]. Rockville (MD): Agency for Healthcare Research and Quality (US); 2013 Sep. Defining Patient Registries and Research Networks. https://www.ncbi.nlm.nih.gov/books/NBK164514/



Opportunities

Challenges

Patient-centered data on…

Disease burden

Patient journey

Unmet medical need

Patient preferences

Natural history of disease

Subgroups

Outcomes and endpoints

Acceptability

Precedence

Standardization

Quality

Validity

Heterogeneity

FasterCures. From Anecdotal to Actionable: A Case for Patient Perspective Data. Available from: http://www.fastercures.org/assets/Uploads/PDF/From-Anecdotal-to-Actionable.pdf

Patient engagement activities to develop recommendations

All disease-modifying therapies | *Fingolimod*

ICER (2017)	CADTH (2011)	NICE (2012)
• Patient / patient advocate interviews • Survey facilitated by the MS Coalition (nearly 16,000 participants)	• Patient group responded to common drug review call for patient input	Two patient experts: • First two Committee discussions • Submitted written evidence

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	ICER (2017)	CADTH (2011)	NICE (2012)
Disease / treatment burden	• Health plan restrictions • Risk of PML • Out-of-pocket costs • Route of administration • Dosing frequency • Risk of side effects • Monitoring / blood tests	• Fatigue • Difficulty walking • Memory or attention difficulty • Numbness or tingling • Pain • Bladder problems • Depression	• Loss of independence • Implications for employment • Impact on emotional wellbeing, which can lead to depression.
Patient preferences	Some patients prefer oral delivery, others equally comfortable with injections	Oral delivery preferred over injections	Oral delivery preferred over injections
Outcomes and endpoints	Long-term outcomes • Independence • Delay disability • Prevent relapse new MRI lesions		

Identifying PPRs

- ISPOR Digest of Databases is being updated and will include patient registries
  https://www.ispor.org/sigs/Digest-of-International-Databases.asp
- Agency for Healthcare Research and Quality’s Registry of Patient Registries
  https://patientregistry.ahrq.gov/
- Patient registries in Europe
  http://parent-ror.eu/#/registries?page=2

Accelerated Cure project for multiple sclerosis

Feinstein Kean Healthcare
An Ogilvy Company

ASU

iConquerMS™

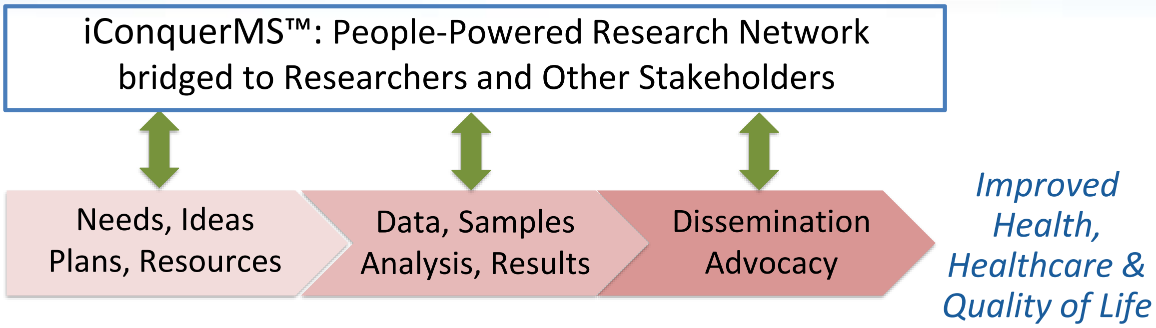
The MS People-Powered Research Network

To improve health, healthcare, and quality of life for people with MS by connecting those affected by MS, caregivers, clinicians, and researchers, and to work together to accelerate innovation, research, and the application of new knowledge.

YOU?

iConquerMS

iConquerMS™ - www.iConquerMS.org



iConquerMS™: People-Powered Research Network bridged to Researchers and Other Stakeholders

Needs, Ideas
Plans, Resources

Data, Samples
Analysis, Results

Dissemination
Advocacy

Improved Health, Healthcare & Quality of Life

- 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

Survey	**REAL MS™ Status**
Demographics	Initial, Summer 2016, Winter 2017, Summer 2017
MS Characteristics	Initial, Summer 2016, Winter 2017, Summer 2017
Neuro-QoL Adult Short Form (now called "Quality of Life")	Initial, Summer 2016, Winter 2017✪, Summer 2017
PROMIS® Global Health (now called "Overall Health")	Initial, Summer 2016, Winter 2017, Summer 2017
Physical Activity	Summer 2016, Winter 2017, Summer 2017
Other (Medical) Conditions	Summer 2016, Winter 2017, Summer 2017
Use of Complementary & Alternative Medicine	Summer 2017

✪ added Bowel, Bladder and Vision from MSQLI

REAL MS™: Research Engagement About Life with MS

What Affects iConquerMS™ Participants Most?

Rank Order	Neuro-QoL Domain (5-point Likert scale questions) Scored: 1[worst], 2, 3, 4 , 5[best]	Average Score (N = ~1,400)
1	Fatigue	2.89
2	Satisfaction with Social Roles and Activities	3.09
3	Sleep Disturbance	3.59
4	Positive Affect and Well Being	3.59
5	Ability to Participate in Social Roles and Activities	3.60
6	Anxiety	3.67
7	Cognitive Function	3.71
8	Emotional and Behavioral Dyscontrol	3.92
9	Lower Extremity Functional Mobility	3.93
10	Depression	4.11
11	Stigma	4.17
12	Communication	4.41
13	Upper Extremity Function Fine Motor ADL	4.54

All respondents as of May 2016

iConquerMS

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The other symptoms and quality of life domains are rarely available in MS clinical trial results and submissions

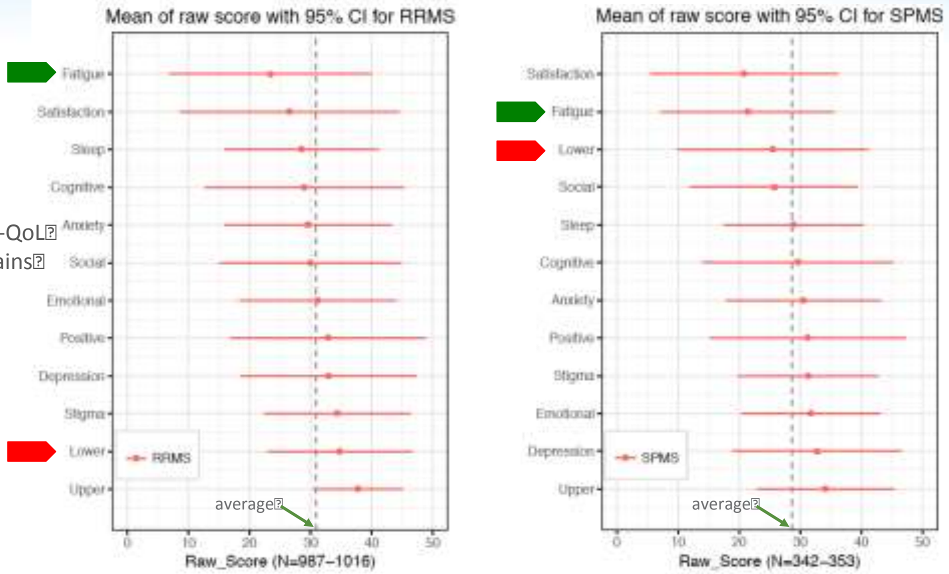
Mobility is captured in the regulatory outcome measure Extended Disability Status Scale

iConquerMS

What Affects iConquerMS™ Participants Most? - More Detail

Details are Important

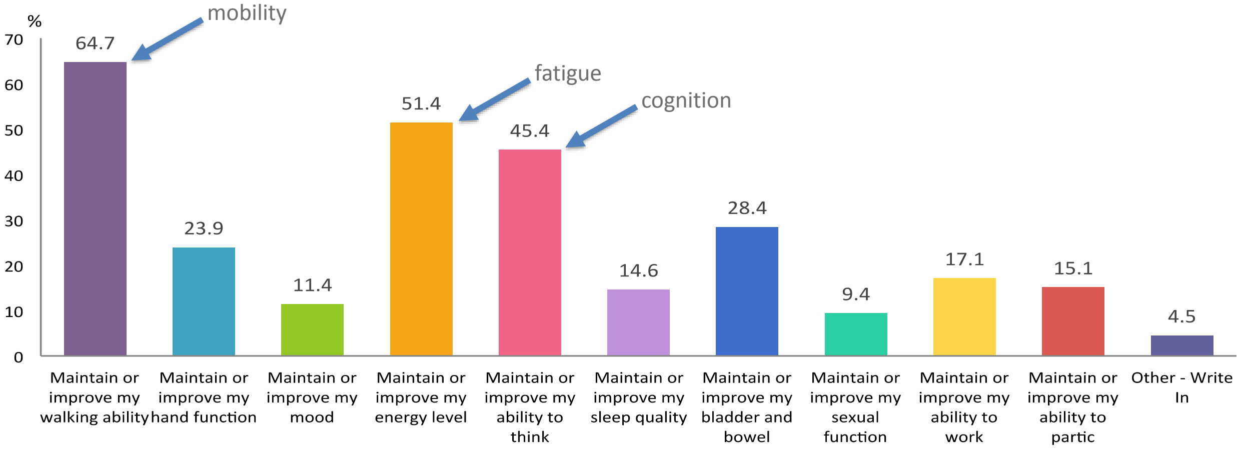
Neuro-QoL domains



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 Guilhaume,
Scientific Project Manager, EUnetHTA JA3
Direction de l'Evaluation 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

DG Santé and CHAFEA

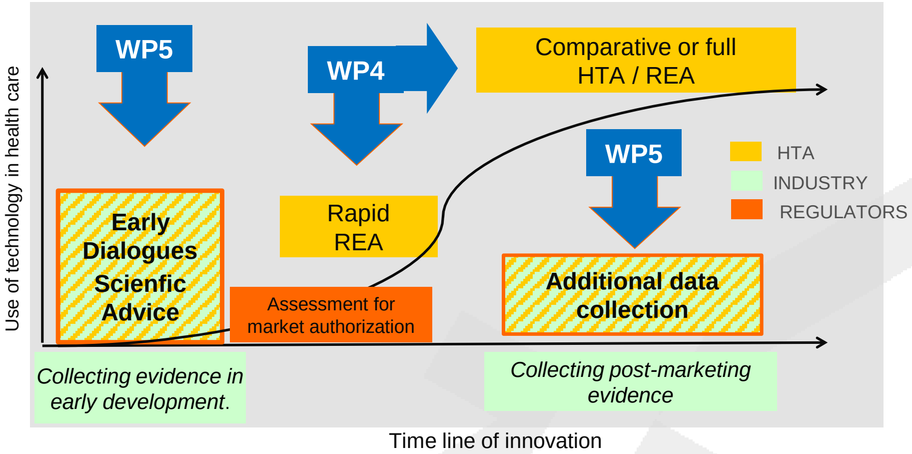
EUnetHTA Assembly

Executive Board

Work package 1 Coordination - Directorate Dutch Health Care Institute

WP 2 Dissemination	WP 3 Evaluation	WP 4 Joint Assessment	**WP 5 Evidence**	WP 6 Quality management	WP 7 Implementation
Lead: ISCIII	Lead: TLV	Lead: NIPHNO Co-lead: LBI ZIN	Lead: HAS Co-lead: GBA	Lead: IQWiG Co-lead: KCE	Lead: NICE Co-lead: Agenas

Patient Voice all along the health technology life-cycle



WP5

WP4

WP5

Comparative or full HTA / REA

🟨 HTA
🟩 INDUSTRY
🟧 REGULATORS

Early Dialogues Scienfic Advice

Rapid REA

Assessment for market authorization

Additional data collection

Collecting evidence in early development.

Collecting post-marketing evidence

Use of technology in health care

Time line of innovation

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:
  - EUnetHTA 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

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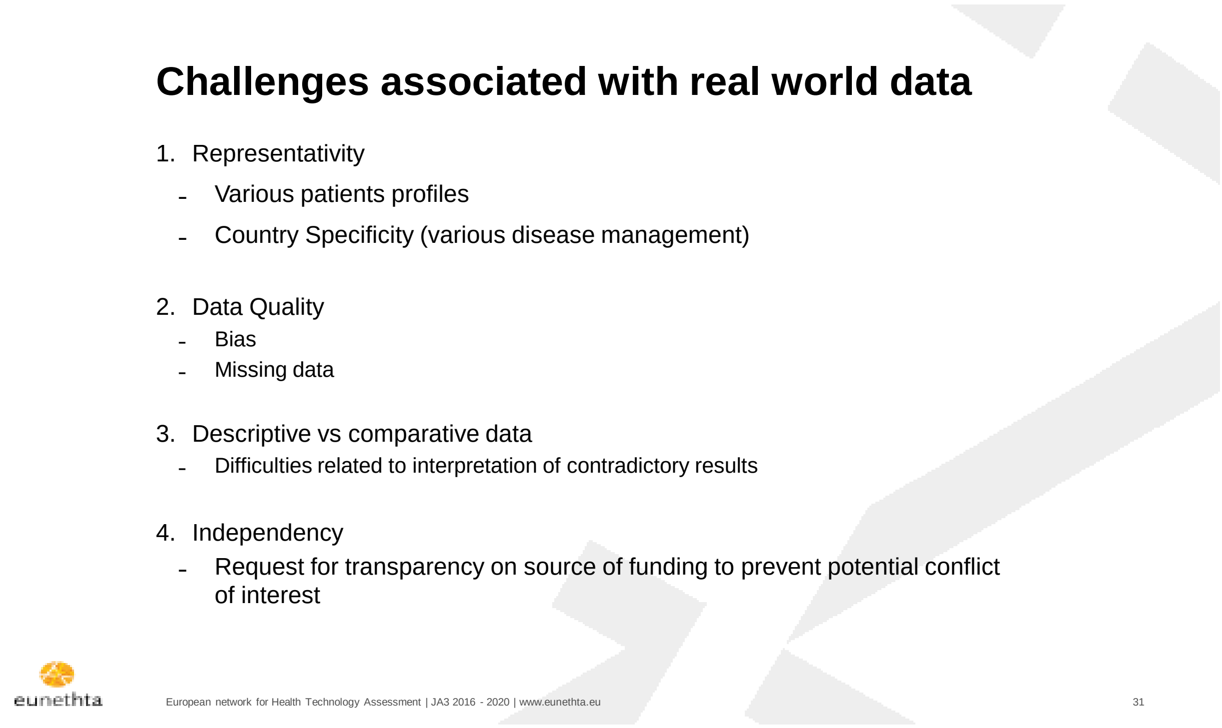
Usage of Patient-based evidence by HTA bodies

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



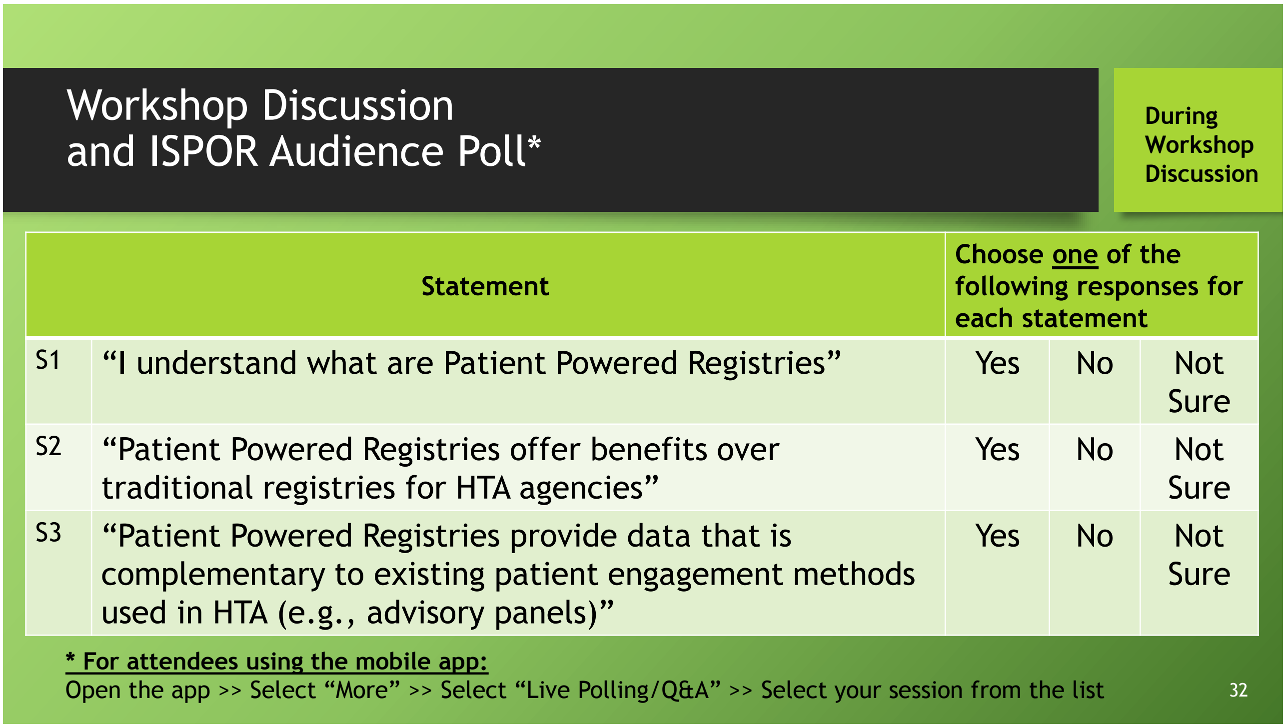
Challenges associated with real world data

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*

During Workshop Discussion

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

<u>* For attendees using the mobile app:</u>
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Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: "I understand what are Patient Powered Registries"

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: “Patient Powered Registries offer benefits over traditional registries for HTA”

Live Content Slide

When playing as a slideshow, this slide will display live content

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

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Continue the discussion on ISPOR app & …..

Workshop Discussion Leaders

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