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SMALL NUMBERS, BIG ISSUES

ISPOR Rare Disease Special Interest Group

November 5, 2019 7:00 to 8:15



www.ispor.org

Moderator:

Sandra Nestler-Parr, PhD, MPhil, MSc, *Head of Market Access, Akcea Therapeutics, London, United Kingdom*

Speakers:

John Guerino, MHS, *Manager, Scientific and Health Policy Initiatives, ISPOR*
Laura Crippa, *Managing Director, RAREg, Milan, Italy*

Agenda

Topic	Presenter(s)
Welcome and Review of Agenda	Sandra Nestler-Parr
Overview of Special Interest Group Structure	John Guerino
Rare Disease Special Interest Group	Sandra Nestler-Parr Laura Crippa
Next Steps and Q&As	Sandra Nestler-Parr
Sign up	All
Adjournment	

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Overview of Special Interest Group Structure

John Guerino, MHS,
Manager, Scientific and Health Policy Initiatives, ISPOR

Antitrust Compliance Statement

- ISPOR has a policy of strict compliance with both United States, and other applicable international antitrust laws and regulations.
- Antitrust laws prohibit competitors from engaging in actions that could result in an unreasonable restraint of trade.
- ISPOR members must avoid discussing certain topics when they are together, including, proprietary or company-specific prices, fees, rates, profit margins, or other terms or conditions of sale.
- Members have an obligation to terminate any discussion, seek legal counsel's advice, or, if necessary, terminate any meeting if the discussion might be construed to raise antitrust risks.

The Antitrust policy is available on the ISPOR website, under "About ISPOR."

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ISPOR Special Interest Groups

- | | |
|--|-----------------------------------|
| • Biosimilars | • Oncology |
| • Clinical Outcome Assessment | • Open Source Models |
| • Digital Health | • Patient-Centered |
| • Health Preference Research | • Personalized/Precision Medicine |
| • Medical Devices and Diagnostics | • Rare Disease |
| • Medication Adherence and Persistence | • Statistical Methods in HEOR |
| • Nutrition Economics | • Real World Evidence |

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Special Interest Group (SIG) structure



SIG Leadership:

- Chair-elect
- Chair
- Past chair
- Each position is a 1-year term, resulting in a 3 year commitment

Each Working Group is led by a Co-Chair

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Responsibilities of SIG Leadership (Chair, Chair-elect, Past Chair)

- Provide overall direction and leadership
- Identify the topics SIG members would like to address
 - Categorizing topics into long, short term projects
 - Identify the appropriate platforms for delivery
- Work with the Working Group co-chairs to ensure the timeliness of the key project
 - Address any issues within the group
- Provide quarterly updates to the SIG membership
 - Current activities, new developments in field
- Report the progress of the SIG projects
 - Via the yearly business and project plan to HSPC
- Recruit new SIG members

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Working Groups within a SIG

Key Project Working Group:

- Manuscript in Value in Health or Value Outcomes & Spotlight

Member Engagement Working Group:

- Webinars
- Journal Club
- Presentation at ISPOR meetings or other organization meetings
- Encouraged communication



Each working group is led by a Co-Chair

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Responsibilities of SIG Working Group Co-Chairs

Key Project:

- Serve until the project is complete (average 24 months)
- Lead the project
- Ensuring adherence to ISPOR processes and working group deadlines
- Address any conflicts or issues

Member Engagement Projects:

- Serves for at least one year
- Work with SIG chairs to identify member engagement topics
- Identifying speakers, articles, topics for member engagement activities
- Ensuring that all member engagement activities occur

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Those interested in SIG leadership positions, please contact Theresa Tesoro at ttesoro@ispor.org

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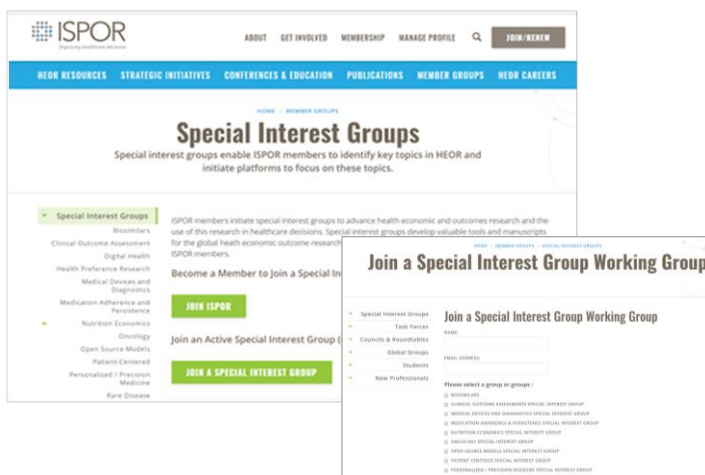
Sign up for a SIG or SIG Working Group



Visit ISPOR webpage www.ispor.org:

- Select “Member Groups”
- Select “Special Interest Groups”
- Click button to “Join A Special Interest Group”
- Complete the form ‘Sign up now’
 - Sign up sheet
 - Provide a business card

Or email: scientificgroups@ispor.org



The screenshot shows the ISPOR website's 'Special Interest Groups' page. It includes a navigation bar with links like 'HOME', 'MEMBER GROUPS', and 'JOIN/RENEW'. The main heading is 'Special Interest Groups' with a subtext: 'Special interest groups enable ISPOR members to identify key topics in HEOR and initiate platforms to focus on these topics.' Below this, there's a list of Special Interest Groups (SIGs) including Biomarkers, Clinical Outcome Assessment, Digital Health, Health Preference Research, Medical Devices and Diagnostics, Medication Adherence and Persistence, Nutrition Economics, Oncology, Open Source Models, Patient-Centered, Personalized / Precision Medicine, and Rare Disease. A 'Join ISPOR' button is visible. To the right, there's a form titled 'Join a Special Interest Group Working Group' with fields for Name, Email Address, and a dropdown to select a group. Below the form, there's a list of groups to choose from, including Biomarkers, Clinical Outcome Assessment, Digital Health, Health Preference Research, Medical Devices and Diagnostics, Medication Adherence and Persistence, Nutrition Economics, Oncology, Open Source Models, Patient-Centered, Personalized / Precision Medicine, and Rare Disease.

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Rare Disease Special Interest Group

Sandra Nestler-Parr, PhD, MPhil, MSc,
Head of Market Access, Akcea Therapeutics

Laura Crippa,
Managing Director, RAREg, Milan, Italy

Rare Disease SIG

Previous Working Group projects:

- 2 publications in *Value in Health*

Membership (Oct 2019):

- 300 members

VALUE IN HEALTH 18 (2015) 906–914



Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.elsevier.com/locate/jval

Health Policy Analysis

Rare Disease Terminology and Definitions—A Systematic Global Review: Report of the ISPOR Rare Disease Special Interest Group

Trevor Richter, PhD, MSc^{1,*}, Sandra Nestler-Parr, PhD, MPhil², Robert Babela, PhD, MSc³, Zeba M. Khan, RPh, PhD⁴, Theresa Tesoro, MSN⁵, Elizabeth Molsen, RN⁶, Dyfrig A. Hughes, PhD, MSc⁶

VALUE IN HEALTH 21 (2018) 493–500



Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.elsevier.com/locate/jval

Articles

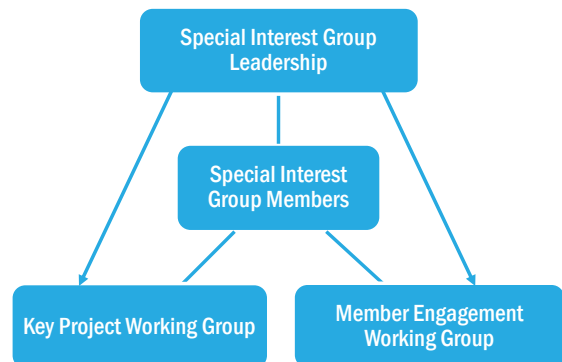
Challenges in Research and Health Technology Assessment of Rare Disease Technologies: Report of the ISPOR Rare Disease Special Interest Group

Sandra Nestler-Parr, PhD, MPhil, MSc¹, Daria Korchagina, PhD, MSc^{2,*}, Mondher Toumi, MD, MSc, PhD³, Chris L. Pashos, PhD⁴, Christopher Blanchette, PhD, MBA⁵, Elizabeth Molsen, RN⁶, Thomas Morel, MSc, MA⁷, Steven Simoens, PhD, MSc, MA⁸, Zoltán Kaló, PhD^{9,*}, Ruediger Gatermann, MA, MBA¹⁰, William Redekop, PhD¹¹

Call for new Rare Disease SIG projects and Working Groups

We are looking for:

- Ideas for new SIG topics
- Projects for the Working Groups
- New SIG members & established SIG members willing to get 'stuck-in'
- A leadership team
- Co-Chairs of the Working Groups
- Your creativity, experience, curiosity, 'hands-on' involvement & commitment to shepherd projects through to the end



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Member Engagement project(s) – ideas for discussion...

ISPOR Book of Terms 2020

- 2003 book includes 100+ entries covering more than 400 terms related to clinical, economic and patient-reported outcomes associated with healthcare, and to the overall management and regulation of healthcare
- Old term monographs need to be reviewed and updated, and new terms to be added
- SIG members volunteer to co-author a term monograph, act as reviewers, or take more responsibility for the overall content as a Term Editor or Section Editor
- Caveat: Deadline March 2020

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LET'S DISCUSS...

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Key project(s) – ideas for discussion...

Do rare/ultra-rare disease treatments require a bespoke HTA process

- Project scope: rare/ultra-rare; global vs Europe vs all countries with established HTA processes; etc.
- Methodology: literature review; policy paper; survey; panel discussion at conference, etc.
- Deliverables/Output: publication; ISPOR conference workshop; etc.
- Timelines: tbd
- Minimum required active contributors: tbd

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LET'S DISCUSS...

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Key project(s) – ideas for discussion...

Budget impact models for rare disease health technologies: where are we?

- Project scope: rare/ultra-rare; Europe vs global vs all countries with established HTA processes; etc.
- Methodology: survey
- Deliverables/Output: publication
- Timelines: tbd
- Minimum required active contributors: tbd

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LET'S DISCUSS...

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Membership / key project(s) – ideas for discussion...

Your suggestions...

- Project scope:
- Methodology:
- Deliverables/Output:
- Timelines:
- Minimum required active contributors:

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LET'S DISCUSS...

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Summary & Next Steps

Head of Market Access, Akcea Therapeutics

Next Steps

- Identify Leadership
- Develop projects, identify working group co-chairs
- Submit overall SIG business plan to ISPOR
 - (NOTE: Key project - manuscript must be approved by Health Science Policy Committee-Science Research Committee)
- Once approved, commence projects
- Solicit members, establish working groups and get to work

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