The challenge, the vision and the value

Meaningful patient involvement in collaborative research projects

Jan Geissler, Patvocates // LeukaNET // CML Advocates Network
IMI impact on patient involvement, 7 Oct 2021
Why should academic researchers, industry and regulators involve patient advocates?

Move away from glossy statements on “patient centricity” towards real involvement of patients in plans, actions, and outcomes

- Gather insights into the day-to-day reality of patients
- Understand “patients’ unmet need”, “patient preferences”, positive “benefit/risk” and “real value” to patients
- Design better clinical trials, services and info resources, leading to wiser investment of limited resources and more meaningful data
Patient involvement in R&D is not a new fashion!
Evolution from 1990s to strong maturity today
Patient involvement in clinical development in practice

Setting research priorities
- gap analysis
- early horizon scanning
- matching unmet needs with research
- defining patient-relevant added value and outcomes

Protocol synopsis
- design
- target population

Protocol design
- relevant endpoints
- patient-reported outcomes/QoL measures
- in-/exclusion criteria
- benefit/risk balance
- ethical issues
- mobility issues/logistics
- data protection
- diagnostic procedures
- adherence measures

Trial Steering Committee
- protocol follow up
- improving access
- adherence

Information to participants
- protocol amendments
- new safety information

Investigator meetings
- trial design
- recruitment
- challenges
- opportunities
- can trigger amendments

Data & Safety Monitoring Committee
- benefit/risk
- drop-out issues
- amendments

Regulatory Affairs
- MAA evaluation
- EPAR summaries
- package leaflets
- updated safety communications
- lay summary of results

Research Priorities

Research Design and Planning
- Patient Information
  - content
  - visual design
  - readability
  - language
  - dissemination
  - contractural issues
  - travel expenses
  - support for family members
  - mobility

Research Conduct and Operations
- Ethics Review
- Informed Consent
  - content
  - visual design
  - readability
  - language

Market Authorization and Post-approval
- Study Reporting
  - summary of interim results
  - dissemination to patient community

- Post-Study Communication
  - contribution to publications
  - dissemination of research results to patient community/professionals

- Health Technology Assessment
  - assessment of value
  - patient-relevant outcomes
  - patient priorities

Fundraising for research

20% of 135 IMI projects had patient organisations in the consortium, 30% in patient advisory boards (60% by 2021)

<table>
<thead>
<tr>
<th>Risk – Benefit Approaches</th>
<th>Patient Organisation</th>
<th>Incorporating Real-World data</th>
<th>Big data in Hematological Malignancies</th>
</tr>
</thead>
<tbody>
<tr>
<td>PROTECT</td>
<td>EPAD</td>
<td>GetReal</td>
<td>LeukaNET + Myeloma Patients Europe + MDS Alliance + Acute Leukemia Advocates Network + Childhood Cancer International + Lymphoma Coalition + CLL Advocates Network</td>
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<td>International Alliance of Patients’ Organisations</td>
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<tr>
<th>Dementia:</th>
<th>Chronic Obstructive Pulmonary Disease (COPD):</th>
<th>Neurodegenerative diseases:</th>
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<td>AETIONOMY</td>
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<th>Autism spectrum disorders:</th>
<th>Recognising adverse drug reactions:</th>
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<td>WEB-RADR</td>
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<td>Asthma UK</td>
<td>European AIDs Treatment Group</td>
<td>European Patients’ Forum</td>
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<tr>
<td>European Federation of Allergy and Asthma</td>
<td>Irish Platform for Patients’ Organisations, Science and Industry</td>
<td>European Patients’ Forum</td>
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<tr>
<td>Lega Italiana Anti Fumo</td>
<td>European Genetic Alliances Network</td>
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<td>Vereeniging Samenwerkende Ouders - en Patiëntenorganisaties</td>
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<td>Genetic Alliance UK</td>
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Source: IMI (2019)
Patient engagement ecosystem has evolved: Trainings and tools

Training for industry
- EUPATI Essentials
- EUPATI Fundamentals
- PFMD PE Training

Training for patient advocates
- EUPATI Patient Expert Training Course
- EUPATI Toolbox
- EUPATI National Platforms

Guidance, Frameworks and tools for patient engagement
- 4 EUPATI Guidances on Patient Engagement
- PARADIGM frameworks
- PFMD how-to-guides

Matchmaking & Best Practice Sharing
- PFMD Synapse
Practical guidance on patient involvement in collaborative research projects

- Checklist when planning patient engagement
- Examples of potential contributions of patients
- Organisational models of patient engagement in research projects
- Identification of the right patient organisation/patient advocates and resourcing the contributions
- Patient involvement plans
- Preparing the patient community for their contribution in the post-application, pre-launch phase

Available for download at
https://www.risingtide-foundation.org/clinical-cancer-research/program/-core-belief
Involvement models for collaborative research projects

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<th>Engagement model</th>
<th>Patient organization’s / patient expert’s role</th>
<th>Impact</th>
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<tr>
<td><strong>Project coordinator, chair or co-chair</strong></td>
<td>Leads and coordinates the research project</td>
<td>Very high</td>
</tr>
<tr>
<td><strong>Steering committee member</strong></td>
<td>Member of the governance board of the research project</td>
<td>Very high</td>
</tr>
<tr>
<td><strong>Work package leader</strong></td>
<td>Coordinates a specific work package in the project</td>
<td>High</td>
</tr>
<tr>
<td><strong>Research project member</strong></td>
<td>Full member of the research teams</td>
<td>Medium</td>
</tr>
<tr>
<td><strong>Patient involvement hub</strong></td>
<td>Full project member, coordinating all contributions from the wider patient community</td>
<td>High</td>
</tr>
<tr>
<td><strong>Associated project partner</strong></td>
<td>Partnership agreement with the research project (but usually no funding)</td>
<td>Small</td>
</tr>
<tr>
<td><strong>Advisor / advisory board member</strong></td>
<td>Providing advice in ethics committee, scientific/project advisory board, data safety monitoring board, but no governance or leadership role</td>
<td>Small</td>
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Patient input into HARMONY Core Outcome Sets, HARMONY Delphi Surveys, HARMONY Research proposals

HARMONY Patient Organisations Masterclass, HARMONY Communications about Big Data in Blood Cancer and about project results to patients and general audience

HARMONY Ethical Framework, De-identification Mechanisms

Patient input into core HARMONY tasks:
• Stakeholder Forum
• Clinical Value Framework
• Access Evidence Framework

The first and largest Public-Private Partnership for Big Data in Hematology
All essential ingredients for patient engagement for more inclusiveness are here

- Shared purpose and **collaborative spirit**
- **Political and institutional will**
- Engagement **frameworks**
- **Capacity** to engage e.g. by patient organisations, companies and institutions
All essential ingredients are here. Let’s engage!

Jan Geissler
jan@patvocates.net