



# Engaging patients in translational research

Karen Ritchie, Jenny Harbour, Lorna Thompson  
Healthcare Improvement Scotland



# WHAT IS TRANSLATIONAL RESEARCH?

- *“In a medical research context, it aims to “translate” findings in fundamental research into medical practice and meaningful health outcomes.”* (Wikipedia)
- Activities which aim to bring healthcare evidence into practice
- *“Research aimed at enhancing the adoption of best practices in the community.”* (National Institutes for Health)
- Includes clinical guideline development, health technology assessment and medicines approval

**What do you think are the most important principles for engaging with RD patients, carers and families?**



## Principles for engagement with rare disease patients, carers, families and organisations: guidance for researchers, policy makers and healthcare providers

- Good practice principles
- Engagement in translational activity\* which aims to bring healthcare evidence into practice

\*clinical guideline development, health technology assessment and medicines approval

1. Systematic searches of the published literature (including EURORDIS literature review)
2. Discussion with key informants with expertise in engaging with rare disease patients and patient organisations to identify key resources and develop draft principles.
3. Consultation with RBP partners on a draft set of principles



- Best practice for patient engagement is **as relevant** to rare disease patients as for those with more common diseases
- Good engagement practices include:
  - providing training; transparent roles and expectations; using plain non-scientific language; realistic timeframes; reimbursing expenses; providing feedback
- Very little published material on engaging rare disease patients in evidence translation activities

## Three themes identified:

- Good examples of patient engagement practices are starting to emerge
- But inconsistency and fragmentation remain the norm
- To ensure a genuine patient-centred model, multi-disciplinary cooperation is needed amongst all stakeholders
- Little expert guidance or consensus exists on who should be involved in drug development, etc

## SIX PRINCIPLES

1. **Consider** patients and patient organisations as co-partners
2. **Recognise** patients have very particular expertise to contribute
3. **Trust** that RD patients, families, carers and organisations are often highly motivated to contribute.
4. **Be mindful** of the broader family issues related to the genetic origin of many RD.
5. **Undertake** thoughtful targeting of recruitment
6. **Appreciate** that RD organisations are often small and respect limitations on their time, resources and expertise



# PRINCIPLES FEEDBACK

What is good?

What is not so  
good?

What changes  
would you make?

Karen Ritchie

[karenritchie@nhs.net](mailto:karenritchie@nhs.net)

Jenny Harbour

[jenny.harbour@nhs.net](mailto:jenny.harbour@nhs.net)

## Websites:

- RARE-GAP [www.rbpresearch.eu](http://www.rbpresearch.eu)
- RARE-GUIDELINES [www.rbpguidelines.eu](http://www.rbpguidelines.eu)

