Developing Guidelines for Rare Diseases: Balancing Rigour and Pragmatism

At the end of this course, participants will have knowledge and skills on how to develop pragmatic guidelines for management of rare diseases

This course is focused on improving the care of patients with rare disease and is designed specifically for those involved in supporting this patient group and therefore relates to the theme of 'Why we do what we do'. The development and use of guidelines is a key element of the new European Reference Networks which are designed to improve the quality of care rare disease patients across Europe will receive through the sharing of knowledge and expertise.

Through our involvement with the EU funded RARE-Best Practice project, we have developed an understanding of the particular issues associated with developing guidelines for many rare diseases including: the burden of involvement for small numbers of patients and carers; the paucity of evidence' and, the scarcity of clinical expertise available to contribute to guideline development.

There is a need in the third sector to offer reliable advice to healthcare services and professionals. This is especially difficult when the evidence base and clinical expertise is scarce. This need is reflected in the numerous enquiries to Healthcare Improvement Scotland from patient organisations and others to provide training and support in developing evidence-based guidance. This course would also offer an opportunity to network within these communities to share learning

This course will provide practical support for representatives of patient organisations, clinicians and others with an interest in developing guidelines for rare diseases and will address the challenges of this work.

09.00	**Registration**
09.30	**Overview of development of rare disease guidelines** *Karen Ritchie, Head of Knowledge and Information*
10.00	**Evaluating rare disease guidelines** Practical exercise 1: Using the AGREE instrument *Lorna Thompson, Health Service Researcher*
10.45	**Guideline development: setting questions, identifying evidence and methodologies** *Karen Ritchie*
11.15	**Coffee**
11.30	**Guideline development: considered judgment and consultation** *Lorna Thompson*
12.00	**Effective engagement with patients, service users and carers and developing patient versions of guidelines** *Karen Graham, Patient Involvement Advisor*
12.40	**How patient organisations can be leaders in guideline development** *Avril Kennan, Medical Research Charities Group, Ireland*
13.00	**End**

Course Facilitators

Karen Ritchie, Deputy Director of Evidence/Head of Knowledge and Information, Healthcare Improvement Scotland

Karen originally trained in the biological sciences and worked on lab-based cancer research projects before developing a career in health services research. Karen's currently role is Deputy Director of Evidence. This directorate undertakes health technology assessments, produces SIGN guidelines and develops national standards and indicators to support health and services improve quality. Karen also leads the Knowledge and Information Unit within Healthcare Improvement Scotland which provides information, research and knowledge management support across the organisation. The team undertakes evidence reviews for quality improvement programmes, rapid reviews of clinical and cost effectiveness of healthcare interventions and provides bespoke support to programmes for sharing knowledge and learning through use of a wide range of tools including social media. The team also supports programme evaluation across the organisation. Karen holds an honorary research post at University of Glasgow and is responsible for the development and implementation of the HIS Research Strategy. Recently her team led the work package of the Rare-Best Practices project that developed a repository of guidelines on rare diseases.

Karen Graham, Public Involvement Advisor

Karen Graham has more than 15 years' experience of involving patients, service users, carers and the public in health service design and development and she is currently Public Involvement Advisor at the Scottish Intercollegiate Guidelines Network (SIGN). Karen's career in public involvement started within the voluntary sector where she was responsible for engaging young people in the development of smoking cessation services within the NHS. Karen joined SIGN in 2004 where she developed the patient and public involvement programme significantly. Her work includes the involvement of patients, service users and carers in guidelines; development of patient versions of guidelines; the involvement of patients, service users and carers in implementation and dissemination activity; and sharing best practice at national and international levels. Recently, she has contributed to the DECIDE project (http://www.decide-collaboration.eu) by developing, evaluating and testing strategies to present guideline information to patients and the public. She is actively involved in the Guidelines International Network (GIN) public involvement group (G-I-N Public).

Lorna Thompson, Health Services Researcher

Lorna has an academic background in biological sciences, with an interest in human nutrition and how it relates to health inequalities. She gained experience of public health needs assessment and evaluation of voluntary sector initiatives at the Centre for Health and Social research in Fife before joining the Scottish Intercollegiate Guidelines Network (SIGN) in 2003, where she facilitated the development of more than 15 evidence-based guidelines covering both physical and mental health topics. Lorna is presently a Health Services Researcher with Healthcare Improvement Scotland and works mainly on rapid reviews to support the work of the Scottish Health Technologies Group (SHTG).

Avril Kennan, CEO, Medical Research Charities Group

Avril has a PhD in genetics and many years subsequent lab experience working on human genetic conditions. She moved from the lab in 2007 to become a patient advocate with DEBRA Ireland. In her role there, as Head of Research and Advocacy, she led many international

initiatives including the development of clinical practice guidelines. She is now CEO of the Medical Research Charities Group in Ireland. In her role there she is passionate about supporting medical research charities in all their research activities and promoting their role in society. Avril has sat on many Boards and Committees, including the DEBRA International Executive Committee, the Rare Diseases Ireland Board and the Irish Health Research Forum Steering Group.

Target audience - Clinicians and representatives of patient organisations and others with an interest in developing guidelines for rare diseases. Maximum number of participants: 24