



## **Deliverable D4.2 - Collection and evaluation of existing guidelines and research recommendations #1**

**Platform for sharing best practices for management of rare diseases**

***(RARE-Bestpractices)***

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**Reference WP(s)**

2 – Platform infrastructure  
4 – Collection development

**Funding Scheme**

Coordination and Support Actions  
(COORDINATING)

**Grant Agreement**

FP7-HEALTH-2012-Innovation-1-305690

**Coordinator**

Istituto Superiore di Sanità (IT)

**Time**

01 January 2013 – 31 December 2016

## Contents

1	Introduction.....	3
2	Workshop format.....	3
3	Utility of the AGREE II instrument for rare diseases .....	3
4	Including appraisals in the guideline database.....	4
	Annex 1 .....	5
	Annex 2 .....	6

## **1 Introduction**

This is the first of a series of deliverables associated with the collection of rare disease guidelines. It reports the preparatory work for the appraisal of the methodological quality of the guidelines in the collection.

A workshop for RARE-Bestpractices project partners associated with workpackage 4 (WP4) was held in Edinburgh, Scotland on 16/17 October 2014 to assess the utility of the AGREE II instrument in evaluation of the methodological quality of rare disease (RD) guidelines.

The AGREE tool is used internationally for the appraisal of clinical practice guidelines. The tool itself, the accompanying manual and instructional materials are available from: <http://www.agreetrust.org/about-the-agree-enterprise/>

There were 14 participants. A list of the participants and organisations represented in the workshop is displayed in Annex 1

## **2 Workshop format**

Participants worked in small groups to conduct AGREE II appraisals of two sample guidelines as below:

Armstrong MJ, Miyasaki JM. Evidence-based guideline: pharmacologic treatment of chorea in Huntington disease: report of the guideline development subcommittee of the American Academy of Neurology. *Neurology*. 2012 Aug 7;79(6):597-603. <http://www.neurology.org/content/79/6/597.long>

Brotherton A, Campos L, Rowell A, Zoia V, Simpson SA, Rae D. Nutritional management of individuals with Huntington's disease: nutritional guidelines. *Neurodegen Dis Manage*. 2012;2(1):33-43. <http://www.futuremedicine.com/doi/pdf/10.2217/nmt.11.69>

Feedback from each group generated discussion around the utility of the AGREE II instrument for rare disease guidelines and on the value and practicality of securing and including appraisals within the RARE-Bestpractices guideline database.

## **3 Utility of the AGREE II instrument for rare diseases**

The group clearly expressed the view that the role of high quality guidelines as tools for healthcare improvement is as relevant to rare as it is to common conditions and that standards should not be lowered for RD.

The following points were highlighted by participants:

- Participation of patients/patient representative organisations is vital.
- Inter-rater reliability appears to depend on interpretation of the question (eg 1vs7 score given despite appraisers having the same viewpoint).
- Judgement 'generosity' can be influenced by mood and context of appraisal workload.
- Information in the guidelines is not always easy to find so appraisal can be time consuming.
- Knowledge of the condition can influence the rating given. A clinical subject expert may identify deficiencies not apparent to non experts.

Participants concluded that the AGREE II instrument is appropriate for evaluation of rare disease guidelines but that some additional guidance for those undertaking the appraisals be developed.

An initial version of this additional guidance is displayed in Annex 2.

#### **4 Including appraisals in the RARE-Bestpractices guidelines database**

Participants agreed the following principles:

- All guidelines which meet the inclusion criteria (see collection development procedure manual Table 3) will be included in the collection irrespective of their methodological quality.
- The guideline record should go live as soon as possible after the systematic search is completed.
- At this stage it will be marked 'waiting for appraisal'.
- As appraisals are completed the full appraisal will sit alongside the record with the overall quality response (1/7-7/7) appearing in the main record.

Potential procedures for recruiting independent guideline appraisers and ways of making the task of value to them were explored but no decisions were finalised on this aspect of the project.

## Annex 1

Participants in the Guideline Evaluation Workshop
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Avril Keenan	DEBRA International	Vienna, Austria
Liesbeth Siderius	The European Academy of Paediatrics	Bruxelles, Belgium
Mathieu Boudes	EURORDIS, European Organisation for Rare Diseases	Paris, France
Mar Trujillo-Martin	Fundación Canaria de Investigación y Salud	Las Palmas de Gran Canaria, Spain
Jenny Harbour Michele Hilton Boon Karen Ritchie Lorna Thompson	Healthcare Improvement Scotland	Glasgow, UK
Paula Bray	Institute for neuroscience and muscle research, University of Sydney	Sydney, Australia
Paola Laricchiuta Christina Morciano	Istituto Superiore di Sanità	Rome, Italy
Henk van Kranen	University of Maastricht	Maastricht, The Netherland
Merryn Pearce	Murdoch Children's Research Institute	Melbourne, Australia
Antonio Atalaia	Newcastle University Upon Tyne	Newcastle, UK

## **Annex 2**

### **Guidance on use of the AGREE II instrument for guideline quality evaluation in Rare Diseases**

#### **General considerations**

- If an item for a particular guideline is considered to be 'not applicable' the item should be scored as '1'.

#### **Scope and purpose (Items 1-3)**

- Rare disease guidelines should be able to address all of the items concerned with scope and purpose.

#### **Stakeholder Involvement (Items 4-6)**

- Although it is likely that one professional group may dominate; comprehensive stakeholder involvement is as important to the development of guidelines for rare diseases as it is for common diseases.
- Scoring of these items should recognise this principle and reflect the extent to which the guideline addresses each item.

#### **Rigour of Development (Items 10-14)**

- The AGREE II quality rating does not depend on the quantity of published evidence but on the rigour of the systematic methods used to identify, select and synthesise evidence and the transparency with which the guideline development group report how they reached recommendations.
- For item 13 (external review by experts) – the experts should include patients, carers, and/or patient groups.

#### **Clarity of Presentation (Items 15-17)**

- When scoring item 16 there may not be a range of options for management of the (rare) condition or health issue. In this case the item would be considered 'not applicable' and scored as '1'.

#### **Applicability (Items 18-21)**

- The extent to which a guideline can provide information on potential facilitators to guideline implementation and describe resource implications may be limited for rare disease guidelines where the implementation setting is likely to encompass diverse healthcare contexts.
- Items should be scored as to how well these issues are addressed.
- The information provided may be country-specific, healthcare system-specific, or generic.

### **Editorial Independence (Items 22-23)**

- For many rare diseases there are likely to be only a small number of experts worldwide. This may limit the potential for editorial independence. Scores should reflect how this was addressed.

### **Overall guideline assessment**

- Before selecting 'yes with modifications', consider whether resources are available to modify the guideline and any copyright issues.
- The existence of only a few or only one guideline on a topic should not prevent a judgment of 'no' on question 2 as it is worthwhile to indicate that better quality guidelines are needed.

### **Notes section**

- Indicate if the guideline is the only (known) guideline on available on the topic.
- Indicate any research recommendations which the guideline identifies.