



RB-DEL.6.3

Newsletter 03

Platform for sharing best practices for management of rare diseases

(RARE-Bestpractices)

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1 Third issue of the RARE-Bestpractices newsletter: the new format

2 Document History

1. Third issue of the newsletter: new format

The third issue of the newsletter has been developed in a new format to permit an easier editing of the text. The text can be directly written in the private section of the website in “ad hoc” fields. Moreover the newsletter will be now archived in the home page of the project website in html format.

The document is organised in two different fields: news and events (see figure 1). The news are also published in the news and events section of the website homepage (see figure 2).

Partner EURORDIS, HIS, LSE and ISS have contributed to the news of this third issue.



www.rarebestpractices.eu

RARE-Bestpractices Newsletter n°3 - December 2014



Value assessment of orphan drugs

Health Technology Assessment (HTA) is playing an increasingly crucial role in the process of appraising and reimbursing medical technologies across Europe. While HTA generally relies on a robust estimation of the clinical cost-effectiveness of a new technology...

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RARE-Bestpractices AGREE II workshop

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2nd IRDiRC Conference Shenzhen

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Engaging with patients

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RARE-Bestpractices welcomes new participants

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Events

5 January 2015

Abstract Submission Opens
G4N Conference "Engaging all stakeholders. Guidelines from a societal perspective"
[go to event](#)

19 -20 March 2015

Rome, Italy
3rd RARE-Bestpractices Annual Meeting
[go to event](#)

29 -30 May 2015

Madrid, Spain
EURORDIS Membership Meeting
[go to event](#)



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Figure 1

Figure 2

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November 25, 2014

Value assessment of orphan drugs



Health Technology Assessment (HTA) is playing an increasingly crucial role in the process of appraising and reimbursing medical technologies across Europe. While HTA generally relies on a robust estimation of the clinical cost-effectiveness of a new technology, the clinical and economic evidence

required for this purpose is often not available for orphan drugs, partly because of challenges related to the recruitment of patients in clinical trials.

In the RARE-Bestpractices project the London School of Economics (LSE) is responsible of mapping out the policies currently in place in selected EU countries on orphan drugs appraisals and exploring the implications these policies have for coverage decisions. A review of the available literature and jurisdiction-specific guidelines on the value assessment of orphan drugs was performed and presented in a peer-reviewed article (*RARE DISEASES AND ORPHAN DRUGS. An International Journal of Public Health*, Vol.1, No.3). The ongoing research is directed to understand how appraisals are conducted, the critical factors leading to positive and negative recommendations and how do assessments feed into clinical practice guidelines. For this reason, 17 case studies on 17 drug-indication pairs are being conducted based on appraisal documents and publicly available guidance from England, France, Germany, Italy, Poland, Scotland, Spain and Sweden (under Work Package 5).

Based on the evidence collected, LSE will explore best practices and share these across EU Member States.

Read here the full article about the LSE study:

<http://rarejournal.org/rarejournal/article/view/60>

Panos Kanavos and Victoria Tzouma

LSE Health, London School of Economics and Political Science

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1. Document History

Date	Author	Changes
01/12/2014	Cristina Morciano	First Draft