



Developing patient information to support decision making regarding rare diseases: **the role of HTA**

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Background. Information for patients with Rare Diseases (RD)



- **Patients with RD** have special needs.
- **Scarce information, complex and difficult to understand:** additional burden (1,2).
- **Professionals:** acknowledge the need and complexity of guaranteeing an exchange of adequate information (3).
- **Healthcare Policy:**
 - **Communication from the European Commission on RD (2008) and the European Council Recommendation on an action in the field of RD (2009).**
 - **Spain: Strategy for Rare Diseases of the NHS (2009, updated in 2014)**



Education and information: key areas for action
Patients play an important role in developing information on RD



(1) Experiences of Rare Diseases: An Insight from Patients and Families. 2010, <http://www.raredisease.org.uk/documents/RDUK-Family-Report.pdf>

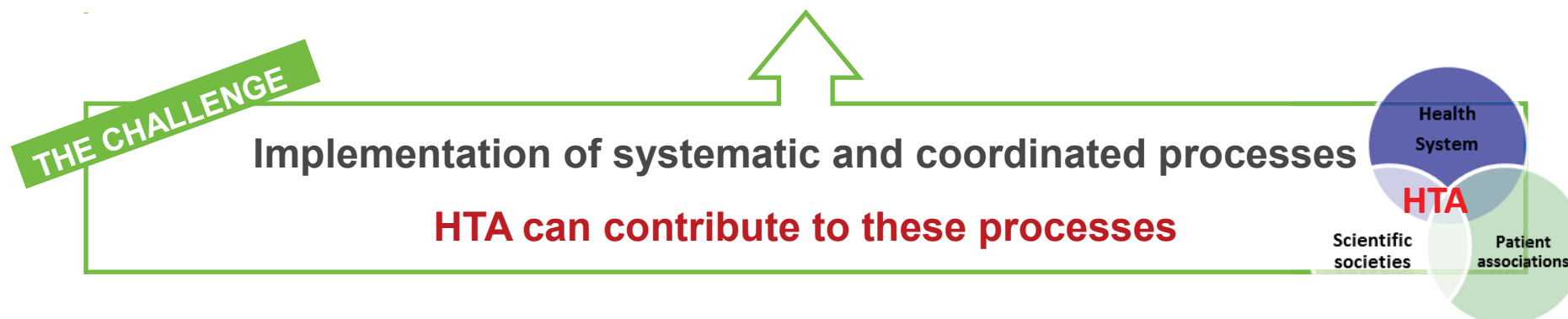
(2) Houyez F, Sanchez de Vega R, Brignol TN, Mazzucato M, Polizzi A. A European network of email and telephone help lines providing information and support on rare diseases: results from a 1-month activity survey. *Interact J Med Res.* 2014;3:e9.

(3) Rare Disease Impact Report: Insights from patients and the medical community, 2013, <http://www.shire.com/shireplc/dlibrary/documents/RareDiseaseImpactReportforWeb.pdf>

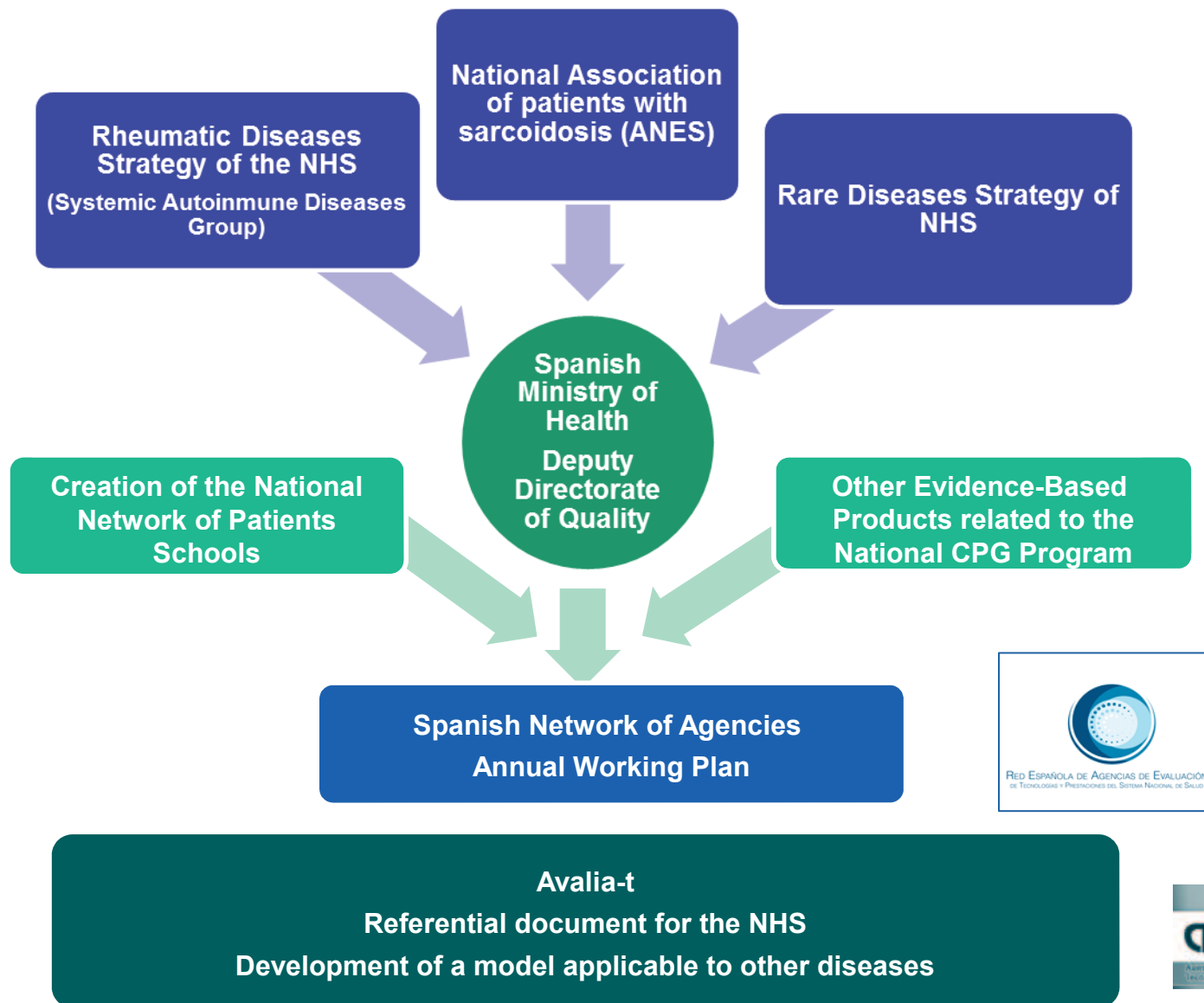
Information for patients with Rare Diseases

Main challenges

- **Providing Quality information:**
 - Based on the best evidence available
 - Incorporate values and preferences of patients
 - Integration of knowledge from experts
- **New technologies facilitate access to information and interaction between professionals and patients.**
- **Integration of the information in the healthcare process:**
 - Strengthen the information provided by professionals
 - Prerequisite for informed decision making
- **To assess the impact on health status, behavioural changes, the patient's experience**



Development of a framework for evidence-based patient information for RD



General description of the project and people involved

- Developed in 5 phases:



1) Analysis of the quality of information documents for patients with sarcoidosis available on the Internet.



2) Identification of the information needs in the care process expressed by the patients.



3) Systematic review of available scientific evidence.



4) Development and design of the document 5) External review



PARTICIPANTS

- Avalia-t:** 2 Senior technicians, 1 documentalist
- Patient Association:** 24 patients and 4 family members
- Clinical Experts:** 7 (from main specialties involved)
- Coordination and monitoring framework:** Ministry of Health, Guiasalud and avalia-t's Head.

2 technicians
2 Patients
2 Clinical Experts

Working Group

Phase 1: What information is available to patients on the Internet?

- **Search:** Google and Google Scholar, other [EBM resources, different HTA Agency websites and other institutions.]
- **Methodological quality:** DISCERN instrument, 16 items organised into three sections (**reliability**, **quality of information** about **treatment options** and **overall quality**):
- **Perceived quality:** patient opinions.

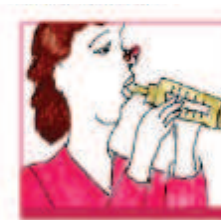
Main results

- 9 materials evaluated.
- Average DISCERN rating= 32.18; 7 of them with ratings of less than 40 (out of a total of 80).
- No document met the expectations of patients: absence of information on the **impact of sarcoidosis on daily life** and **recommendations related to healthy living habits, treatments, prevention and symptoms.**

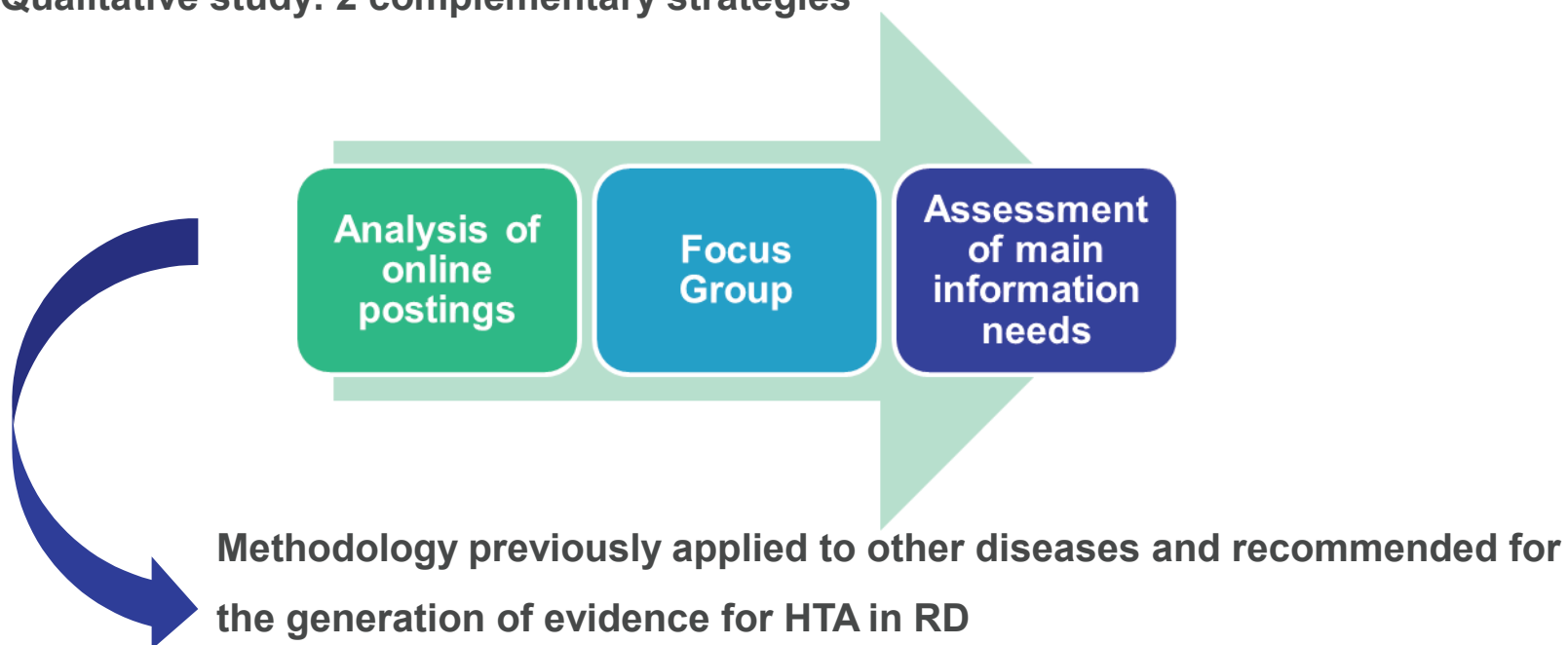
“No information on the impact of sarcoidosis was collected...”

“Information about treatment, prevention, symptoms, etc. was missing.”

Phase 2: What do Sarcoidosis patients need to know? (I)



- **Objective:** To **evaluate the explicit and implicit information needs** of patients with sarcoidosis
- **Qualitative study: 2 complementary strategies**



e. g. Colombo C, Mosconi P, Confalonieri P, Baroni I, Traversa S, Hill SJ, et al. Web search behavior and information needs of people with multiple sclerosis: focus group study and analysis of online postings. *Interact J Med Res.* 2014;3(3):e12; Facey K, Granados A, Guyatt G, Kent A, Shah N, van der Wilt GJ, et al. Generating Health Technology Assessment Evidence for rare diseases. *Int J Technol Assess Health Care.* 2014;30(4):1-7.

Phase 2: Analysis of questions from the patient forum (II)



- **Patient association (ANES) online forum:** since 2012
- April 2014: 268 people were registered (patients and family members).
- **1,200 posts** reviewed and **81 explicit questions** located by 2 technicians + 4 patients.
- **Thematic analysis:**



Themes	Questions
Sarcoidosis	<i>Why am I sick? Am I going to get better? Is it contagious?</i>
Symptoms	<i>Why am I always tired? Why do my joints hurt and feel tingling?</i>
Diagnosis	<i>What are pulmonary function tests and used? Is it always necessary to do a biopsy?</i>
Healthcare assistance	<i>Which is the most appropriate specialist to treat me? When should I go to the emergency room?</i>
Treatment	<i>Why has my doctor not given me any treatment? Is prednisone a immunosuppressant ?</i>
Complications	<i>If I take steroids I can have osteoporosis? Should I take calcium supplements?</i>
Living with sarcoidosis	<i>Should I be vaccinated against any disease? Can I continue playing sports? Can any diet help?</i>
Pregnancy, contraception and menopause	<i>I have fertility problems, is it because of the sarcoidosis? How will sarcoidosis and the treatment affect my hormones?</i>
Family	<i>How do I tell my family that I have sarcoidosis?</i>

Phase 2: Focus group (III)

Participants: 6 women and 2 men (41-57 years old), from different regions, chronic sarcoidosis (n=6).



- **Explicit information needs:** causes, characterization, symptoms & treatment, recommendations about lifestyle and self-management.



“I don’t know anything, I just know about sarcoidosis because I have it”

“I want to know what’s happening to me, and I want to know the consequences it can have for me to start preparing myself”

“I got home completely broken down because I didn’t know what sarcoidosis even was...”

- **Implicit information needs:** the impact of sarcoidosis, perceived problems with health services, treatment and communication with professionals.



“My life has changed a lot since it all started. Now I can’t do many things... I can’t climb stairs or make any physical effort ...”

“They told me I had sarcoidosis. And without explaining anything else, you go home...”

“They gave me more steroids and never explained any alternatives, not in the first or second outbreak. They give you a prescription and that’s it...”

Phase 3: Systematic Review of the evidence



- **Bibliographic search:** CRD databases, Cochrane Library, Tripdatabase, Medline (Pubmed) and Embase (Ovid). This search was conducted without any time limit until October 2014.
- **Inclusion criteria:** systematic reviews, CPG, expert consensus/narrative reviews.
- **Quality of evidence:** Assessment of Multiple Systematic Reviews (AMSTAR) checklist .

Main results

- **Systematic reviews (11):** diagnosis (5), treatment (4), quality of life (1) and cancer(1).

AMSTAR average 7.18 (between 4-9, with a maximum of 11).

- **Expert consensus/narrative reviews (6)**
- **No CPG**

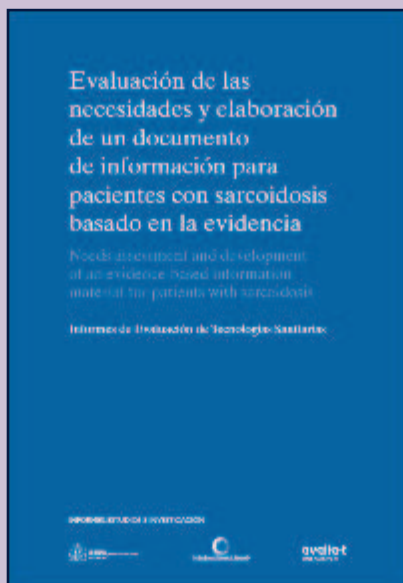
Overall quality of the evidence limited: observational studies with small and heterogeneous samples.

Phases 4 and 5: Document development and external review



- **Working methodology:** face-to-face meetings, online work and teleconferences.
- **Flexible and iterative process:**
 - Guarantee through different perspectives that the information was as complete as possible, and address **current state of scientific knowledge, clinical practice** and **answer the main identified topics**.
- **Development of content:**
 - ✓ **Systematic methodology**
 - ✓ **Current quality standards** for reporting information to citizens:
 - » Content,
 - » Linguistic readability,
 - » Typographic legibility,
 - » Versions: complete version and a leaflet.
- **External Review:** patients and professional

Products



Professionals



Patients



Added value of the products

- **Patients:**
 - **Involved in all phases of the project:** target information to specific needs, ensure comprehensibility.
 - **Patient Associations:** communicated constraints, knowledge derived from shared experience, main concerns and complaints.
- **Qualitative research:** outline the needs, preferences, values and expectations of patients.
- **Experts:**
 - Aspects considered good clinical practice.
- **Evidence:**
 - Adaptation of the current state of scientific knowledge, areas with a low level or absence of evidence: uncertainties have been explained.
- **Dissemination:** active involvement of **professionals and patients** in the dissemination and adoption of the document.



Conclusions



- **Methodology and procedures:** were appropriate and efficient in order to include **patient experiences** and **expert knowledge** in the development of **evidence-based information for Rare Diseases**.
- The document incorporates the **needs of patients** and may **promote the process of shared decision making**, and the necessary **exchange of opinions between patients and professionals**.
- **New challenges:** assessing the applicability of this methodological framework to different scenarios (e.g. lower prevalence, absence of patient associations, reduced availability of treatment options...).

HTA...

... **Can contribute to the empowerment of patients with Rare Diseases:** evidence-based and patient-centered health information.

...We need to **continue advancing with new formulas** that allow the development of different **HTA products for RD**.

Thank you very much for your attention



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