EXPERTS CONSENSUS ON ACTIONS FOR ENHANCING HEALTHCARE AND ACCESS TO ORPHAN DRUGS IN SPAIN

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BACKGROUND

- Although rare diseases (RD) affect a limited number of persons1, the number of disorders that match this definition account for more than 5,0002, estimating that about 20 million people in the EU and nearly 3 million of people in Spain have a RD 3.
- RDs are an important public health issue not only for the clinical and therapeutic complexity they represent, but also because of the little knowledge on their usual trajectory4.
- In Spain the decentralization of health services to the 17 Autonomous Communities has led to a disparity in the way and speed patients access to care and treatments.

OBJECTIVE

This study aims to reach consensus amongst experts on the most feasible actions to be undertaken to facilitate orphan drugs availability and patients’ access to specialized care in the public health sector in Spain.

METHODS

- Two rounds on-line Delphi survey was conducted.
- The 1st round questionnaire was developed based on a review of the literature and two focus groups (Figure 1). It included 32 issues (56 statements); orphan drugs price and reimbursement (5 issues), access to specialist care and to orphan drugs (9 issues), registries (7 issues), and care models for RDs management (1 issues).
- The 2nd round questionnaire only included statements for which consensus was not reached in the 1st round.
- All statements assessed the participants’ desire (D) and prognosis (P) to take place over the next 5 years, based on a 5-point Likert scale (1=total disagreement with; 5=total agreement with).
- Consensus was reached when 75% participants, or more, choose the totally or partially agreed options (agreement), or the totally or partially disagreed alternatives (disagreement).

RESULTS

- Panel consisted in 82 experts (50.9% response rate), including health care managers (n=13), clinicians (n=21), pediatricians (n=20), hospital pharmacists (n=16), patients (n=9), and pharmaceuticals representatives (n=3).
- Agreement was reached in 66.07% (n=37) statements.
- Main results related to public health concerns are presented.

Reference teams

- Consensus was achieved on D and P (Figure 2) related to reference team (a multidisciplinary team with medical and health management background);
  - Reference teams would develop patients’ referral protocols and clinical practice guidelines recognized countrywide;
  - Reference teams would have well defined criteria to make decisions for a better use of orphan drugs;
  - Reference teams would apply tools to clearly inform patients about the potential benefits of treatments and to help them reach shared decisions in clinical practice.

Figure 2. Level of agreement on reference team issues

- Consensus was reached on D and P on the following: the price of orphan drugs would be reviewed if their clinical effectiveness becomes uncertain (D: 79.27%; P: 91.03%) or their target population increases substantially (D: 85.37%; P: 85.90%).

Figure 3. Methods

Aspects to be considered to define reimbursement conditions

- Consensus was reached in the D and in the P that 8 aspects would be considered to define the conditions of reimbursement of orphan drugs (Figure 3):

  1. The disease based registry allows to collect data on the clinical and therapeutic evolution of rare disease patients.
  2. The coordination and integration of the newly with the already available information would be possible due to the creation of a disease based registry.
  3. Serve to generate scientific evidence on the effectiveness of treatments as well as on the burden of diseases.

Figure 4. Level of agreement on aspects to considered to define reimbursement

Registries

- According to a disease based registry, consensus was accomplished on D and P (Figure 4):
  1. The newly generated with the already available information would be integrated;
  2. It would allow to collect data on the clinical and therapeutic evolution of patients; and
  3. It would serve to generate scientific evidence on the effectiveness of treatments as well as on the burden of diseases.

CONCLUSIONS

In order to enhance RD patients care most feasible agreed actions relate to OD access, referral teams creation and registries organization. As a public health issue, this consensus may foster similar discussions in other European countries.