INTRODUCTION

- Rare diseases (RD) affect a limited number of persons (≤12,000 individuals in the EU). However, more than 5,000 disorders match with the definition of RD. As a result, rare disease patients comprise 6% to 8% of the EU population and nearly 3 million people are affected by a RD in Spain.
- The Spanish national health system offers almost universal coverage and a high quality of health care. But, the decentralization of health services to the country’s 17 autonomous communities has led to a variety of management models and a high degree of unevenness in drugs affordability decision making and medical practice. These variations have rendered disparity in the way and speed patients access to care and treatments.

OBJECTIVE

To determine the level of consensus that exits amongst experts on the most likely actions to be implemented to equal access to Orphan Drugs (OD) and specialized care to RD patients’ in the public health sector in Spain.

METHODS

Study Scientific Committee
- 7 major stakeholders in pediatrics, internal medicine, orphan drug evaluation, rare disease registries, patients, and the pharmaceutical industry representatives formed the Scientific Committee who oversaw the development of the study.
- Study design
  - The panelists were nominated by the Scientific Committee or were identified through the main Scientific Societies.
- 2 on-line Delphi rounds were conducted.
  - The content of the 1st round questionnaire relied on a review of the literature and two focus groups. It included 32 issues (56 statements) grouped into 4 sets of information: orphan drugs price and reimbursement (5 issues); access to specialist care and to orphan drugs (9 issues); registries (7 issues) and care models for rare diseases management (1 issue). The 2nd round questionnaire included all 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 year, based on a 5-point Likert scale (1=in total disagreement with; 5=in total agreement with).

Consensus definition
- Consensus was reached when ≥75% participants chose the totally or partially agreed options (agreement), or the totally or partially disagreed alternatives (disagreement).
- Descriptive statistics were applied to determine consensus and quantify its degree. Percentage of panelists with the same response to the same statement was calculated for the 1st and the 2nd rounds.

RESULTS

Delphi panel
- A total of 161 candidates were invited to take part in the Delphi study: 82 participated in the 1st round (50.9% response rate) and 78 in the 2nd one.
- Panelists included health care managers (n=13), clinicians (n=21), pediatricians (n=20), hospital pharmacists (n=16), patients (n=19) and pharmaceutical representatives (n=3) (Figure 1).

Figure 1. Delphi participants

- Consensus only for the D but not for P of occurrence was achieved for 18 (32.14%) statements, showing that participants were less optimistic about the feasible and realistic implementation for these actions in a 5 years time horizon.
- Most panelists clearly agreed on the D (90.24% agreed) for reviewing OD reimbursement conditions every 5 years, based on the results obtained from usual clinical practice. However, regarding prognosis there was no consensus on this issue (74.36% agreed).

Figure 2. One evaluation of orphan drugs

- Although it was desired (95.06% agreed) that only one evaluation of OD would be conducted instead of the several currently required at the regional health authorities level, only a very small proportion of panelists (15.38% agreed) perceived its implementation feasible over the next 5 years in Spain (Figure 2).

Figure 3. A reference team of medical specialists

- It was highly desired (98.78% agreed) that a reference team composed by medical specialists, synchronized with local care teams, would coordinate patients’ treatment and care. However, more than half of participants (56.41% disagreed) were not optimistic regarding its realization in the near future (Figure 3).

Figure 4. Role of home care teams

- Consensus was reached regarding the role of home care teams. Their intervention would imply a more efficient management of patients with RD, contributing to decrease the frequency of hospital visits and admissions, as well as improving patients’ health related quality of life (91.46% agreed). Nevertheless, many respondents (56.41%) disagreed about its implementation in the near future (Figure 4).

CONCLUSIONS

Although perceived as highly needed, RD experts are little optimistic about the immediate future of endeavors that require the review of health care processes to succeed in easing access to OD and specialized care for RD in Spain.

REFERENCES