



Access to treatment: unequal care for European rare disease patients

A Rare Barometer survey

February 2017



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1. Introduction

In 1999, the European Union regulation on orphan medicinal products stated “**patients suffering from rare conditions should be entitled to the same quality of treatment as other patients**”.

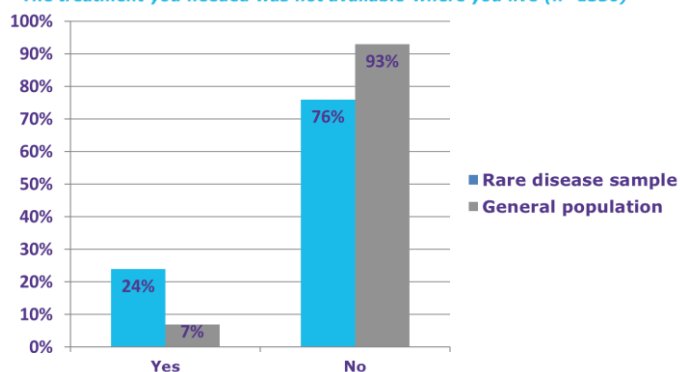
Today, the issue of access to medical treatments is under pressure due to a number of recent developments including the economic crisis, leading to falling healthcare spending in many Member States, and an ageing population. In another EURORDIS survey carried out among the rare disease population, **44% of respondents reported a worsening of their access to care** in general over the last two years¹. In this same study, difficulties in accessing treatment were reported to be mainly due to the organisation of care (for 47% of respondents) including long waiting times (21%), difficulties identifying a specialised doctor (15%), or long distance to visit doctor (11%). Financial obstacles were a second explanation, accounting for 29% of all causes, with high costs and poor reimbursement (22%), or advance payment with lengthy reimbursement (7%).

As we already know, only a very small number of rare diseases have curative treatment, but what is the level of access for existing rare disease treatments, either symptomatic or disease-modifying? Does it really differ from the general population? Rare Barometer participants (people affected by a rare disease who answer regularly to EURORDIS surveys) were asked about their access to treatments:

2. Results of the survey (Question: *During the past 12 months did it ever happen that you did not get the medical treatment you needed because...*)

24% of the rare disease sample declare that during the past 12 months they did not get the medical treatment they needed because **the treatment was not available in their country** (see methodology, point 2 at the end of the document). The **difference with the general population is striking: 17% higher** (in percentage points, see methodology point 3). The gap between the general population and rare disease patients was reported as even deeper in some countries such as in the United Kingdom where 31% say that the treatment was unavailable in their country compare to only 3% of the general population.

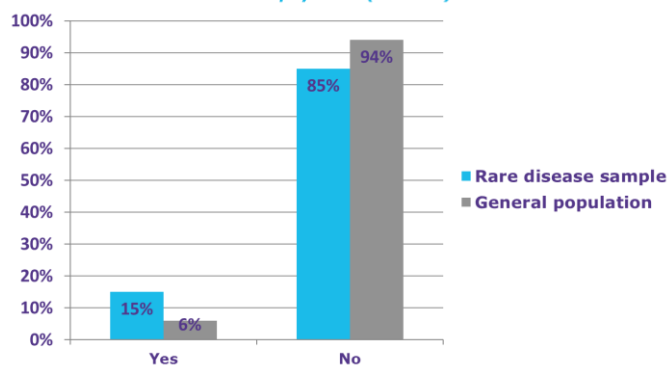
During the past 12 months did it ever happen that you did not get the medical treatment you needed because...
The treatment you needed was not available where you live (n=1350)



¹ EURORDIS Access Campaign Survey 2014-2016, by the Drug Information Transparency and Access (DITA) Task Force, funded by an Operating Grant of the European Commission. 1,943 respondents from 31 European countries. Situation was reported as stable for 42% and improving for 8% over the last two years. <http://www.eurordis.org/access-campaign>

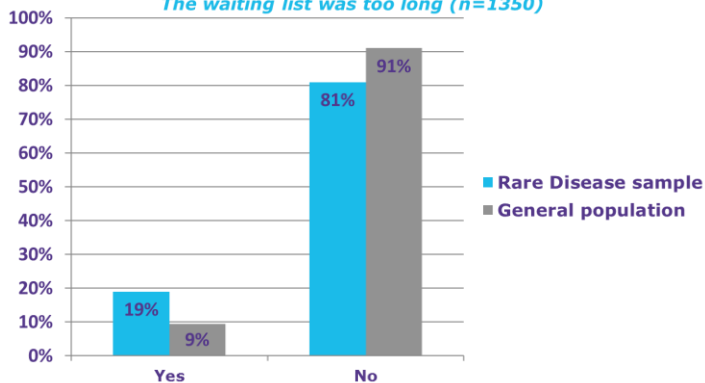
When asked if they **did not get the medical treatment they needed because they were not able to pay for it, 15% of the respondent answered yes.** This represents a difference of 9 percentage points compared to the general population.

**During the past 12 months did it ever happen that you did not get the medical treatment you needed because...
You could not pay for it (n=1350)**



Respondents were then asked **if they were prevented from accessing the treatment they need because the waiting list was too long: 19% answered yes.** There is a marked difference in comparison to the general population: 10% percentage points more. This waiting time is mainly related to the time required to obtain a visit or appropriate hospitalisation.

**During the past 12 months did it ever happen that you did not get the medical treatment you needed because...
The waiting list was too long (n=1350)**



Methodology

1. The survey was carried out through the EURORDIS Rare Barometer Survey Programme;
2. The question referred to medical treatment in general including medicines (disease modifiers and symptomatic treatments), surgery and medical devices;
3. The exact same question taken from the ISSP (International Social Survey) has been asked, a continuing annual programme of cross-national collaboration on surveys covering topics important for social science research. The last wave was carried out in 2011. Comparison are made with the results of the European continent. The category "no" correspond to : No it didn't happened/No need of medical treatment/Non response;
4. Fieldwork started on 11th of February and ended on 16th of February 2017; with a total of 1350 respondents from 21 European Countries;
5. The sample is composed of 54% of patients and 46% of carers, carers answered in relation with the situation of the patient they care for.

If have any questions, please contact Sandra Courbier, Surveys and Social Studies Manager, Rare Barometer Leader: sandra.courbier@eurordis.org



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Thank you to all Rare Barometer partners:



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