

Survey design for rare disease patient organizations

RD-Portugal

**União das Associações das
Doenças Raras de Portugal**

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Structure of the presentation regarding our survey design:

1. Topic
2. Goals
3. Team
4. Resources
5. Methods
6. Recruitment-Dissemination
7. Data analysis
8. How we reported and used the results



1. Topic

Health-Related topic

Patient experience regarding the use of Centers of Expertise for rare diseases in Portugal

2. Goals

- Understand the **patient experience** of people living with a rare disease and their family members in Centers of Expertise in Portugal.
- Use the gathered data to **influence Portuguese Health Authorities** and ensure rare disease Centers of Expertise are tailored and responsive to the needs of people living with a rare disease.
- Use the collected evidence to **guide an interactive workshop**, based on the World Café methodology, to elicit the opinions of several rare disease stakeholders in Portugal on the Centers of Expertise.



3. Team

A winning team



Executive board members
(design; test)

+



Specialized company in patient-centric research
(review)



4. Resources

- Google forms to construct the questionnaire.
- Excel to analyse the data.



Background Research

We did background research from reliable sources:

- asked the DGS (National Authority for Healthcare in Portugal) to give us a capacity building session on the topic.

We obtained background information, from this reliable source, on:

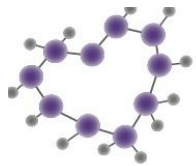
- how many reference centers exist in Portugal in terms of rare diseases/disease groups.
- which hospitals are accredited in Portugal as Centers of Expertise for Rare Diseases/Disease Groups.



5. Writing the questionnaire

I am the mother of a girl with an ultra-rare disease, called Sanfilippo Syndrome:

- diagnosed 13 years ago
- since dx, been dealing with many challenges to obtain appropriate healthcare for my daughter
- president and founder of the Sanfilippo Portugal Association .



Sanfilippo Portugal
sanfilippoportugal.com



Based on my experience in terms of health care at a National IEM Expertise Center, and...

To find similarities with my many challenges, a series of telephone interviews were conducted, with various Sanfilippo families:



IMPORTANT TIP: DIVERSE SAMPLE WAS CHOSEN
(informed about the use of testimonials)

- living in large urban centres **vs.** outside urban centres
- paediatric **vs.** adult age
- different social backgrounds
- different levels of education

The aim was to obtain from the Sanfilippo families, the broadest possible range of testimonies, covering a national perspective.

The interviews were conducted through an informal dialogue, without using a strict questionnaire, to capture the real challenges (qualitative).

**TIP**

To better involve patients/carers and get more detailed information for the initial survey design, telephone interviews work better.



Writing the questionnaire

- open-ended questions were mostly included in the questionnaire but also an open question (to collect more in-depth info from participants): ex: *"can you describe your experience regarding transition from clinical care into adulthood?"*

GDPR compliant

- we did not collect emails
- we did not collect sensitive data: name of disease, age, gender
- only RD-Portugal has access to the individual responses and is the sole responsible for data analysis
- only collective results will be published and shared with others
- a Note was added: *"The answers to this questionnaire are anonymous. The data is kept by RD-Portugal for data analysis purposes and for as long as is necessary to make an aggregate analysis of this questionnaire."*



Reviewing the questionnaire By the team

The questionnaire was reviewed by the team:

- the deadline to be online (we had the World Café event deadline = 206 participants)
- time to complete
- asking the right questions in the right way



6. Recruitment and Dissemination

Carried out via:

- **Email:** to patient organizations that are members of RD-Portugal, asking them to spread the word amongst their members.
- **Social media:** to all rare disease patients, even if they don't have a patient organization represented in RD-Portugal.
- By the members of our **Scientific Advisory Board**, via their networks.





7. Data Analysis



- Marking all answers as 'mandatory': facilitates the task of analysing the data - reduces blanks

The results from google forms:

- Were exported to **Excel**
- The '**blanks**' were eliminated for the real # of answers given *i.e.* check the data for duplicates, and incomplete responses and exclude them.
- The graphics were constructed using *Dynamic charts/graphs*, after this '**data cleaning**'.
- As statistical analysis we did **percentages**.

The analysis of 'closed' answers is directly quantified (example):

" How far do you have to travel from your home to the Expertise Hospital to go to appointments?"

- *Up to 20 kilometers*
- *20 to 50 km*
- *50 to 100 km*
- *more than 100 km"*



7. Data Analysis

The analysis of 'open' (qualitative options) answers should be more careful (example):

"According to your rare disease diagnosis, are you clinically followed up in any of the hospitals that are recognised Expertise Centers for your disease/group of diseases?"

- *Yes, I am treated at the Reference Centre listed for my rare disease*
- *I am not being treated at a Reference Centre for my rare disease, even though it is listed*
- *There is no Expertise Centre for my rare disease (group of diseases)*
- *I am followed up, in a specific appointment for my rare disease*
- ***Other:.....(qualitative - description)"***



Analyse "Other's" answers individually:

- they often fit into the available answers
- Try to group them to analyze if they origin a new quantifiable option.



Leave as few 'open' answers as possible:

- If on the one hand they allow you to obtain more information, they make it difficult to analyse the data to a quantitative questionnaire.



8. How to report and use the results

The results :



- Were **presented at an event organized by RD-Portugal**, in *World Cafe* format, on the theme: **“The Reference Centers for Rare Diseases in Portugal and the close relationship with ERNs”**.

The meeting was attended by various specialized stakeholders in rare diseases in Portugal, *i.e.* patient organization rep.'s, clinicians, researchers, consultants, journalists, industry and national health authority's representatives and a representative from Eurordis. – *kick-off*.

- Will be used **to build a final report to present to the competent National Authorities** :

- as a result of the World Café, a final report will be produced with the conclusions of the meeting.

- Will add very important data about patients **‘real-life experience’** regarding the use of the healthcare system, by rare diseases patients, which will be included into the final report.

- Will be shared with the participants and/or general public: by creating a **social media campaign** sharing the result/highlights in order to give back to those who participated, to acknowledge their participation.

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Obrigada!

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