

HOW SHOULD I ASK?

Survey design for rare disease patient organisation

Jessie Dubief, Social Research Director

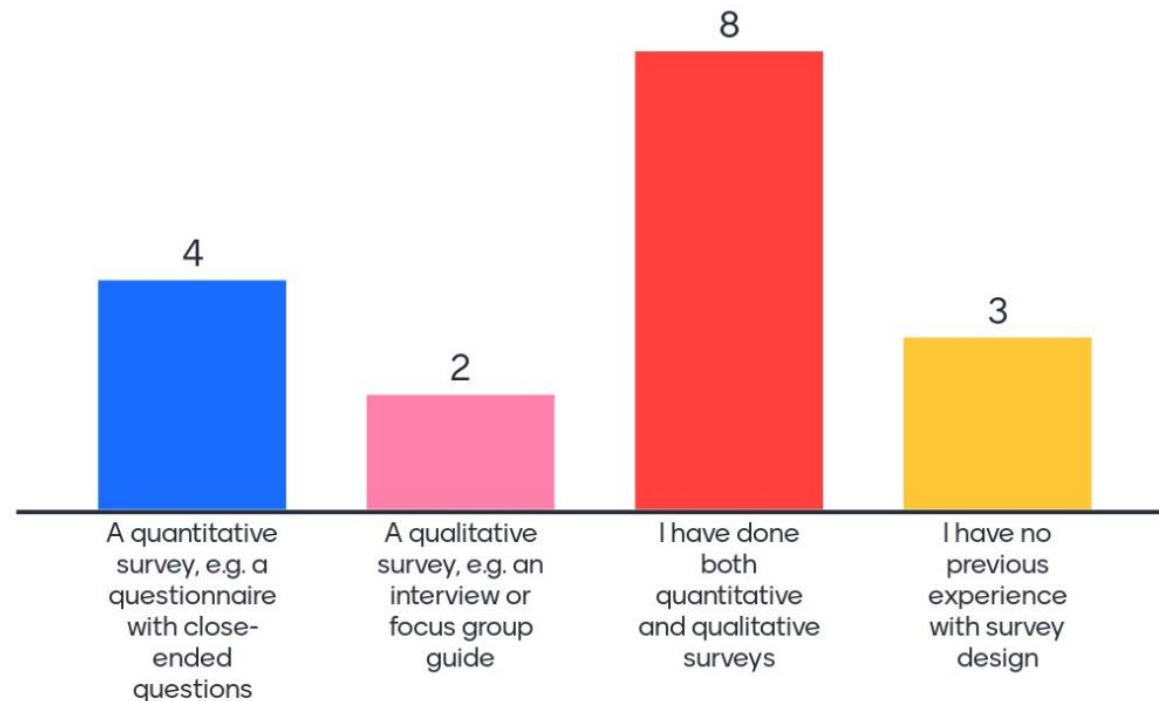
Rita Francisco, Survey Manager



LET'S GET TO KNOW EACH OTHER BETTER!

Some fiery questions about your experience and a quick quiz:

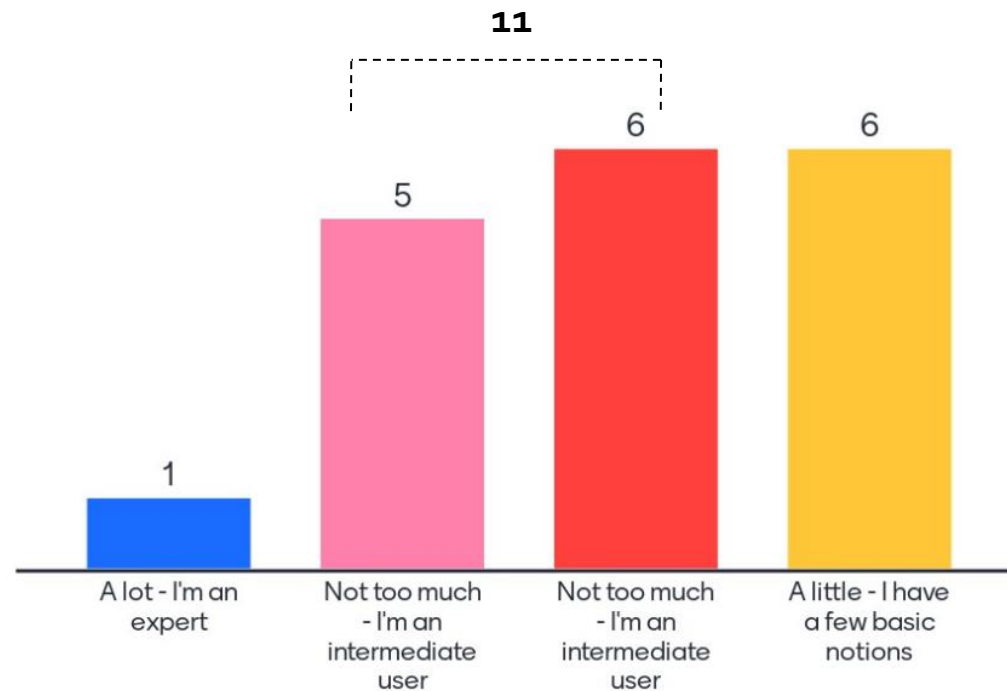
Have you ever designed...?



LET'S GET TO KNOW EACH OTHER BETTER!

Some fiery questions about your experience and a quick quiz:

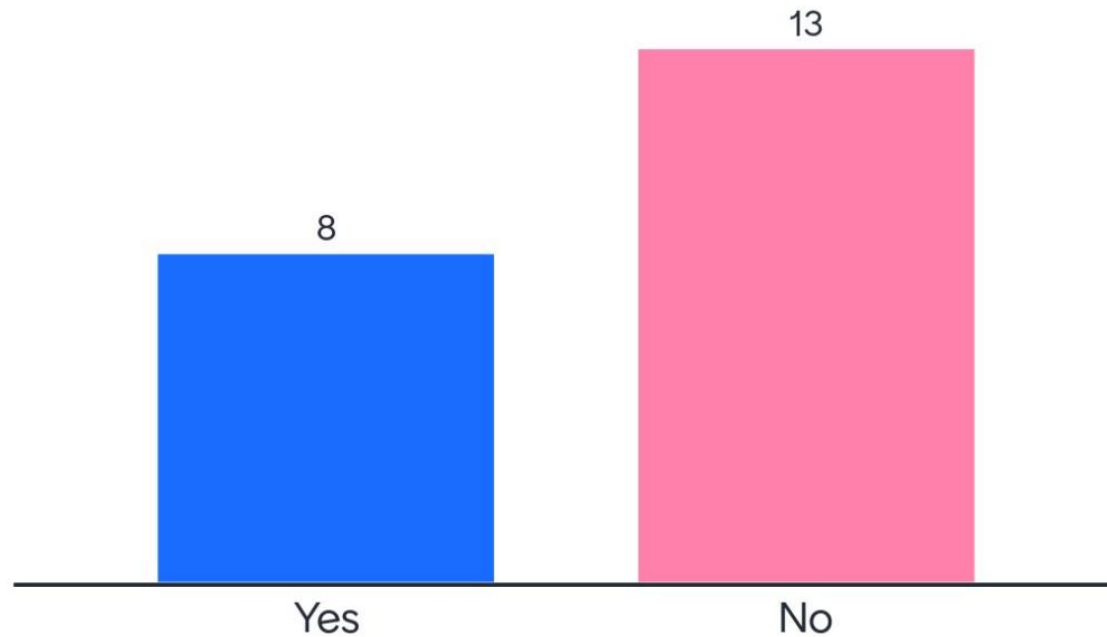
How much do you know about Survey Design?



LET'S GET TO KNOW EACH OTHER BETTER!

Some fiery questions about your experience and a quick quiz:

Are survey and questionnaire synonyms?



IMPORTANT DIFFERENCE!

Questionnaire



Survey

An instrument of data collection that is used to gather information on a given topic (e.g. rare disease patients experiences with healthcare services).

A survey is a process that includes several steps, and the most important is to make sure that each step is relevant to answer your research questions. Hence, a questionnaire can be used as part of a survey, but a survey goes beyond that and can also encompass the use of other data collection tools, such as focus groups or interviews.

SURVEY DESIGN: IT IS A MULTI-STEP PROCESS

Good survey design requires time, planning and resources. **It is a multi-step process:**

1. Preparation

- What do you want to know? For what objectives? Who is your target population? Does the information already exist? Can you join forces? What are your resources?

2. Data collection

- How to best collect information? How to design it well and make it GDPR compliant?

3. Recruitment & dissemination

- How to reach out to respondents? How to follow dissemination?

4. Analysis

- Who analyses the results and how?

5. Making the most out of the results

- How to communicate the results? How and with whom to use results / information?



TOPIC SELECTION AND OBJECTIVE SETTING

“WHAT DO YOU WANT TO EXPLORE?”

Don't jump right into drafting questions! First and foremost, consider carefully what you want to achieve, how and for what.

Policy or advocacy-related topics	Health-related topics	Education-related topics	Research-related topics	Strategic topics
E.g.: The lived experience of searching for a diagnosis for your rare disease.	E.g.: Develop a patient journey; Draft a Clinical Practice Guideline on treatment for your rare disease.	E.g.: Create an educational/capacity-building programme for parents of children living with a rare disease.	E.g. : Set up a registry for your disease; Develop a Patient Reported Outcome Measure (PROM).	Can result from a single or combination of the other topics



TOPIC SELECTION AND OBJECTIVE SETTING

OBJECTIVE SETTING

- **Establish the purpose and intent of your project**, and what you want to achieve in broad terms.
- After you identify your overall objective, you may start to **reflect on what information you need to get there**.

Policy or advocacy-related topics	Health-related topics	Education-related topics	Research-related topics	Strategic topics
<p>E.g.: The lived experience of searching for a diagnosis for your rare disease.</p> <p>Understand the difficulties encountered by people living with your rare disease to get a diagnosis and develop a tailored awareness and advocacy campaign to improve the diagnostic options for your disease.</p>	<p>E.g.: Develop a patient journey; Draft a Clinical Practice Guideline on treatment for your rare disease.</p> <p>Improve the quality of health care for your patient community. Improve access to treatments for your patient group.</p>	<p>E.g.: Create an educational/capacity-building programme for parents of children living with a rare disease.</p> <p>Empower parents of children living with a rare disease to cope with the disease in a holistic manner, including how to navigate the healthcare system and how to access educational and social services.</p>	<p>E.g. : Set up a registry for your disease; Develop a Patient Reported Outcome Measure (PROM).</p> <p>Increase and improve clinical trial recruitment and design of clinical trials for your disease.</p>	<p>Can result from a single or combination of the other topics</p> <p>Set your patient organisation’s priorities for the upcoming months or years based on the needs and priorities of your membership or broader patient community.</p>

SURVEY DESIGN: SEVERAL METHODS & TOOLS TO CHOOSE FROM



WHAT ARE THEY?

Quantitative

Research methods resulting in numbers and figures to better understand a specific topic.

Qualitative

Research methods based on more interactive collection instruments that allow in-depth exploration of individuals' perspectives about a specific topic.

Mixed methods

Multimethod research that uses more than one method of data collection, combining quantitative and qualitative methods. e.g., Delphi process.

TOOLS

Questionnaires:

- They can be administered online on paper, over the phone.
- They can be filled by participants themselves or by the research team.
- Inexpensive tools that can allow to gather data from small & large audiences.

- One to one interview, a single participant is interviewed at a time.
- Focus groups usually of 6 - 8 participants where discussions are led by moderators and ideally observers can be involved to take notes

Questionnaires, interviews, focus groups, etc. used in combination.

WHEN SHALL I USE EACH OF THEM?

Good choice if you are interested in measuring **'how many'** or **'how often'**

Good choice if you are interested in measuring **'why'** or **'how'**

allows to combine the advantages of the measurable conclusions of quantitative data and the in-depth, detailed insights of qualitative data. On the one hand, **"stories"** can provide meaning to **"numbers"**; on the other hand, **"numbers"** can strengthen **"stories"**.

LET'S GET TO KNOW EACH OTHER BETTER!

For you, what are the uses of survey design as a patient representative?

26 responses



RARE DISEASES' SURVEY SPECIFICITIES

- **Sample size** and **population size**
- **Sensitive data:** [article 9](#) of GDPR
- **Anonymity:** when presenting results, make sure that the characteristics you give for your respondents does not allow to recognize them, especially when populations are small (few patients in one country...).
- **Samples are usually not representative:** only talk about respondents for descriptive statistics (do not refer to the whole population) and use significant data for analytical statistics.
- During results analysis and communication, **the subject of your sentences should correspond to your statistical unit:** respondents / patients / carers / people living with rare diseases...
- Respondents are usually contacted through patient organisations, and online: be mindful of the **social characteristics of respondents** (probably more educated, usually more respondents in Western Europe, more engaged...).

CASE STUDY

EURORDIS' SURVEY INITIATIVE

Rare Barometer survey design process





THE RARE BAROMETER PROGRAMME

The survey initiative of EURORDIS-Rare Diseases Europe

eurordis.org/rare-barometer

WHAT



Not-for-profit initiative

WHEN



Created in 2016

WHO



Run independently by EURORDIS-Rare Diseases Europe

WHOM



Collect the experiences and needs of **all people living with a rare disease** and their **family carers** in a robust and systematic way

OBJECTIVE



WHY



Evidence-based advocacy



WHAT TOPICS HAVE WE BEEN FOCUSING ON?

Cross-cutting topics of **interest** and **relevance** to all rare diseases

DAILY LIFE



2017

TREATMENT



2018

HEALTHCARE



2020

FUTURE OF RD



2021

NEWBORN SCREENING



2023



2018

RESEARCH



2019

DATA



2020

COVID-19



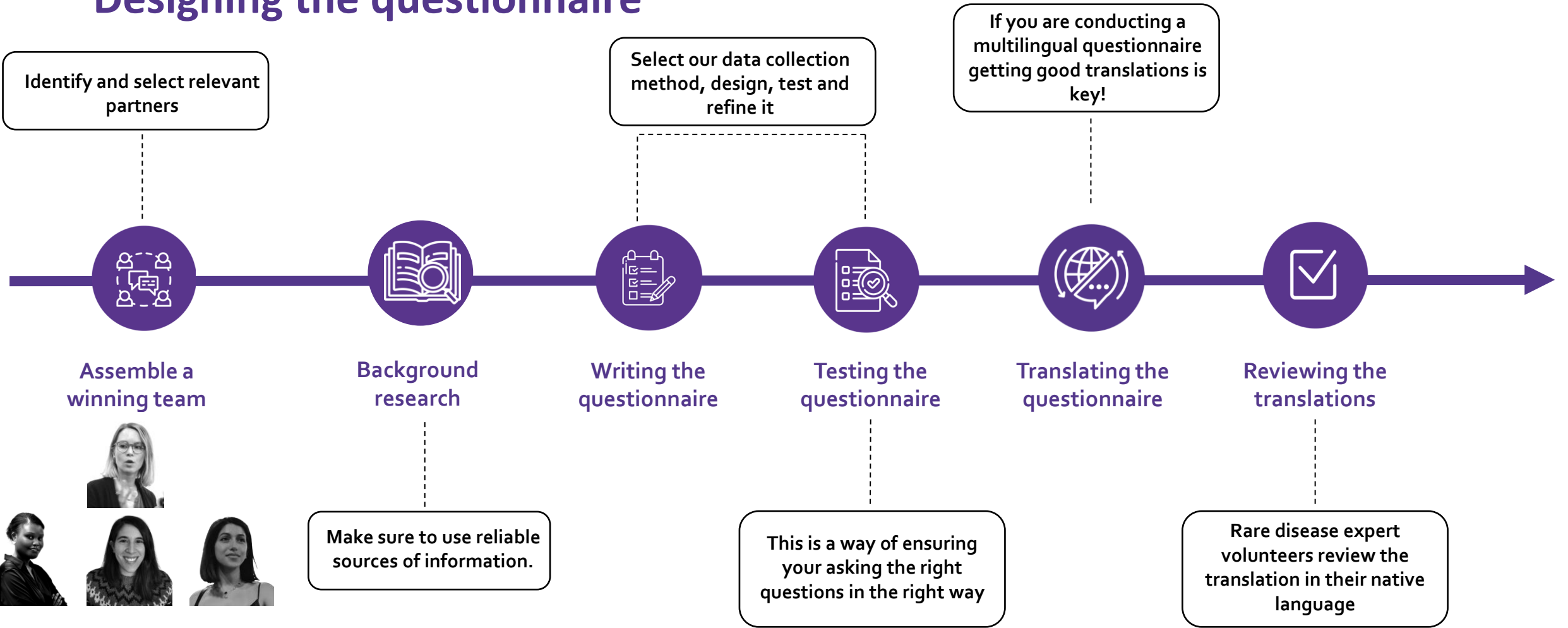
2022

DIAGNOSIS

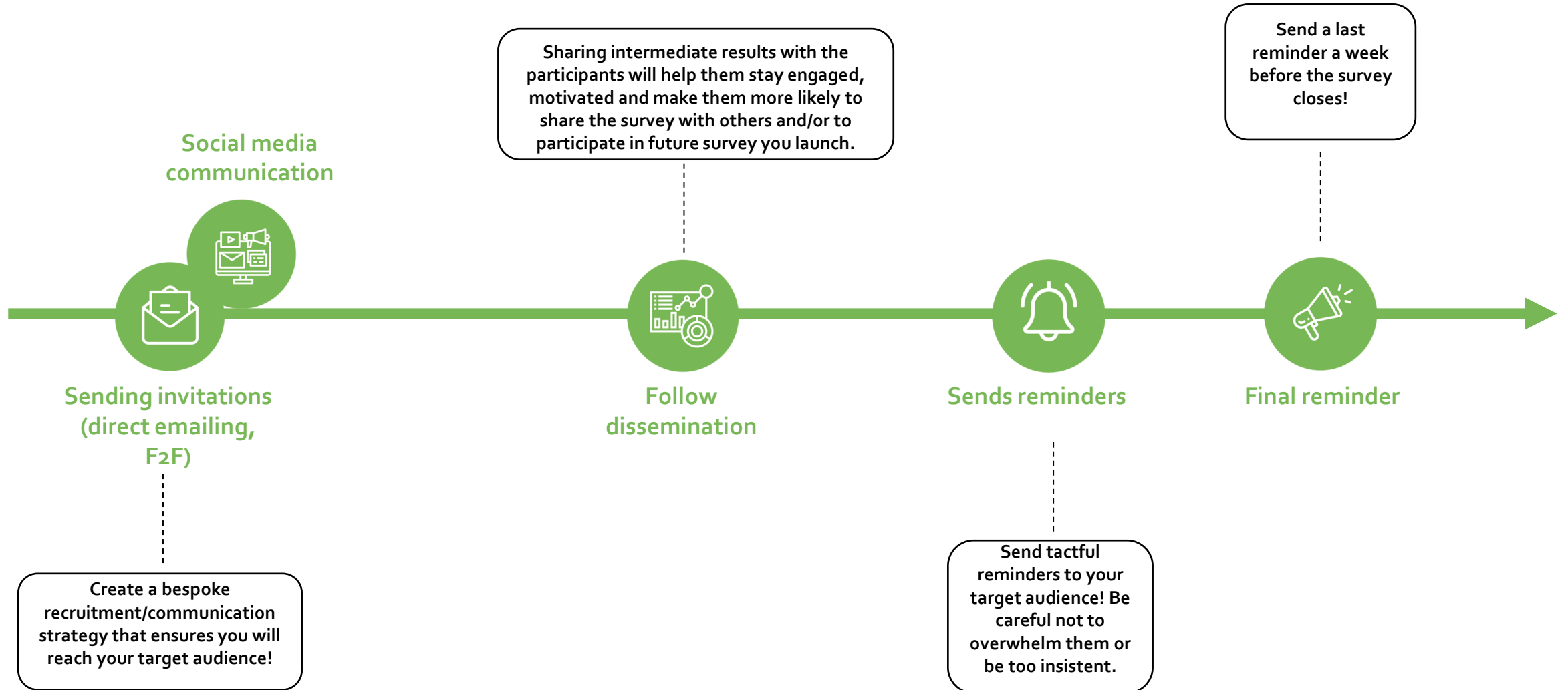


OUR METHODOLOGY

Designing the questionnaire



SURVEY DISSEMINATION





SURVEY DISSEMINATION

Choose the format of your reporting wisely and if possible, tailor the results (content + format) to your target audience to ensure they are as impactful as possible !



Clean and analysis the data



Publish and disseminate your results

Before carrying out any kind of statistical analysis, first check the data for duplicates, and incomplete responses and exclude them. The type of statistical tests you can or should do depends on several factors, including on how you are aiming to publish/disseminate them and on your target audience

Paper
English



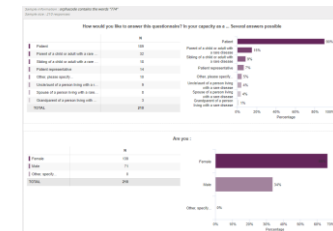
REPORT
English



FACTSHEET
4 pager - 7 languages
+ on demand



DASHBOARD
Each question of the questionnaire
Frequency and percentages
24 languages





Questions?

NEW OPEN ACADEMY COURSE

Survey design for rare disease patient organisations

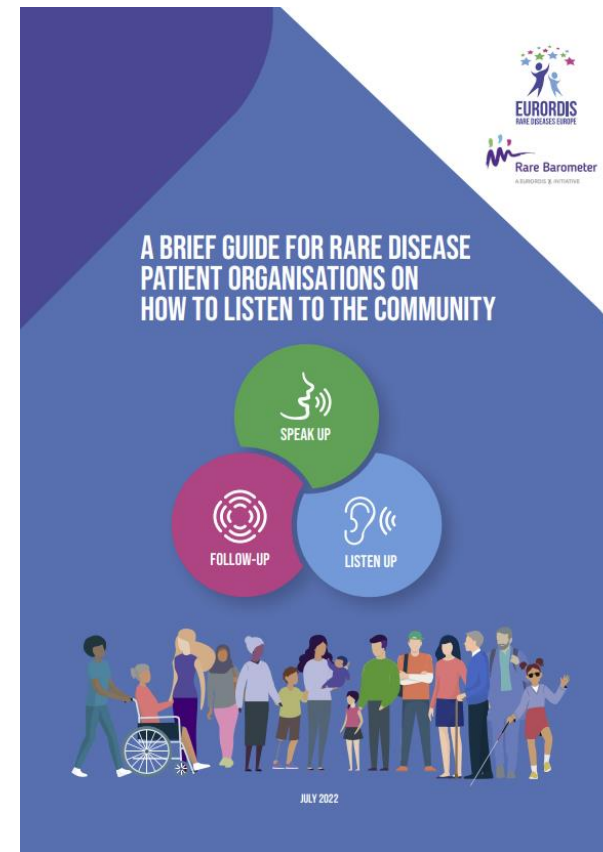


SURVEY DESIGN FOR RARE DISEASE PATIENT ORGANISATIONS

Inspired by:

'Speak up. Listen up. Follow up. A brief guide for rare disease patient organisations on how to listen to the community

tiny.cc/speak_up_listen_up_guide



SURVEY DESIGN FOR RARE DISEASE PATIENT ORGANISATIONS



What it is & where to find it

Practical, step-by-step online course on survey development and implementation.

Freely available on:

openacademy.eurordis.org



Who is it for

Patient representatives
who want to understand how to capture & act on the insights of community
Anyone interested in **developing survey design skills**



When can you take it

There is no fixed deadline!

You can take it at your own pace and in our own time.

Around 3-5 hours to complete



STRUCTURED INTO 8 LESSONS



Each lesson includes:

- tips and case studies



- materials and activities: short videos, guides, actionable tools



- Quiz time!





SURVEY DESIGN FOR RARE DISEASE PATIENT ORGANISATIONS

ONLINE COURSE

Step 1: Define your topic and objectives

☰ Step 1: Define your topic and objectives

📖 Quiz 1 - Knowledge check!

Step 2: Assemble your team

☰ Step 2: Assemble your team

📖 Quiz 2 - Knowledge check!

Step 3: Do your background research

☰ Step 3: Do your background research

📖 Quiz 3 - Knowledge check!

Step 4: Define who you want to survey and how to reach out to them

☰ Step 4: Define who you want to survey and how to reach out to them

📖 Step 4: Tips!

📖 Quiz 4 - Knowledge check!

Step 5: Select your method

☰ Step 5: Select your method

☰ Ethics, informed consent and data protection

📖 Step 5: Tips!

☰ Online survey software tools

📖 Quiz 5 - Knowledge check!



SURVEY DESIGN FOR RARE DISEASE PATIENT ORGANISATIONS

ONLINE COURSE

Step 6: Analyse and interpret your data

- ≡ Step 6: Analyse and interpret your data
- 🔗 Step 6: Tips!
- 🔗 Quiz 6 - Knowledge check!

Step 7: Report and disseminate your results

- ≡ Step 7: Report and disseminate your results
- 🔗 Step 7: Tips!
- 🔗 Quiz 7 - Knowledge check!

Step 8: Put your data into action

- ≡ Step 8: Put your data into action
- ≡ Example 1: Co-creating rare disease care pathways
- ≡ Example 2: Developing patient journeys
- ≡ Example 3: Measuring patient experience with care
- ≡ Example 4: Creating a patient-reported outcome measure
- ≡ Example 5: Co-designing surveys for rare connective tissue diseases
- ≡ Example 6: Using your community's voice to assess rare disease expert centres
- ≡ Example 7: Using Rare Barometer survey results

BREAKOUT GROUPS

Time to hear about
some more real-life
examples



OUR CASE-STUDIES

Breakout group 1

- Rare Diseases Ireland
- National survey
- Qualitative methods
- Topic: Care pathways



Vicky McGrath

Breakout group 2

- RD-Portugal
- National survey
- Quantitative methods (questionnaire)
- Topic: Patient experience with Centres of Expertise



Raquel Marques

WRAP-UP

**Final conclusions
and Q&A**





Questions?



Thank you!

to the Rare Barometer participants,
partners and corporate donors in 2022!

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