



Patient Voices Programme plan

Patient voice Survey Panel



What is the aim of Patient Voices programme ?

Context : Necessity for rare disease patients to be involved in health, research and social decision making

Facilitate and streamline the inclusion of patient perspectives



Provide a high quality evidence base



Support advocacy and policy making activities at EURORDIS



Promote further research on patient perspectives

Which methods are going to be used?



Patient voices' governance



Quantitative surveys are the biggest challenge

- Statistics about rare disease population don't exist (except prevalence)
- Small number of people per disease
- A list of people affected by rare disease doesn't exist

➡ Rare disease population is not considered as a whole

➡ Usual methods for sampling (by quota or random) are not applicable

Sampling rare population by creating a survey panel

- Principle : gathering small raindrop in glass of water
- Used by research institute to carry out quantitative surveys (Kantar, Ipsos...)



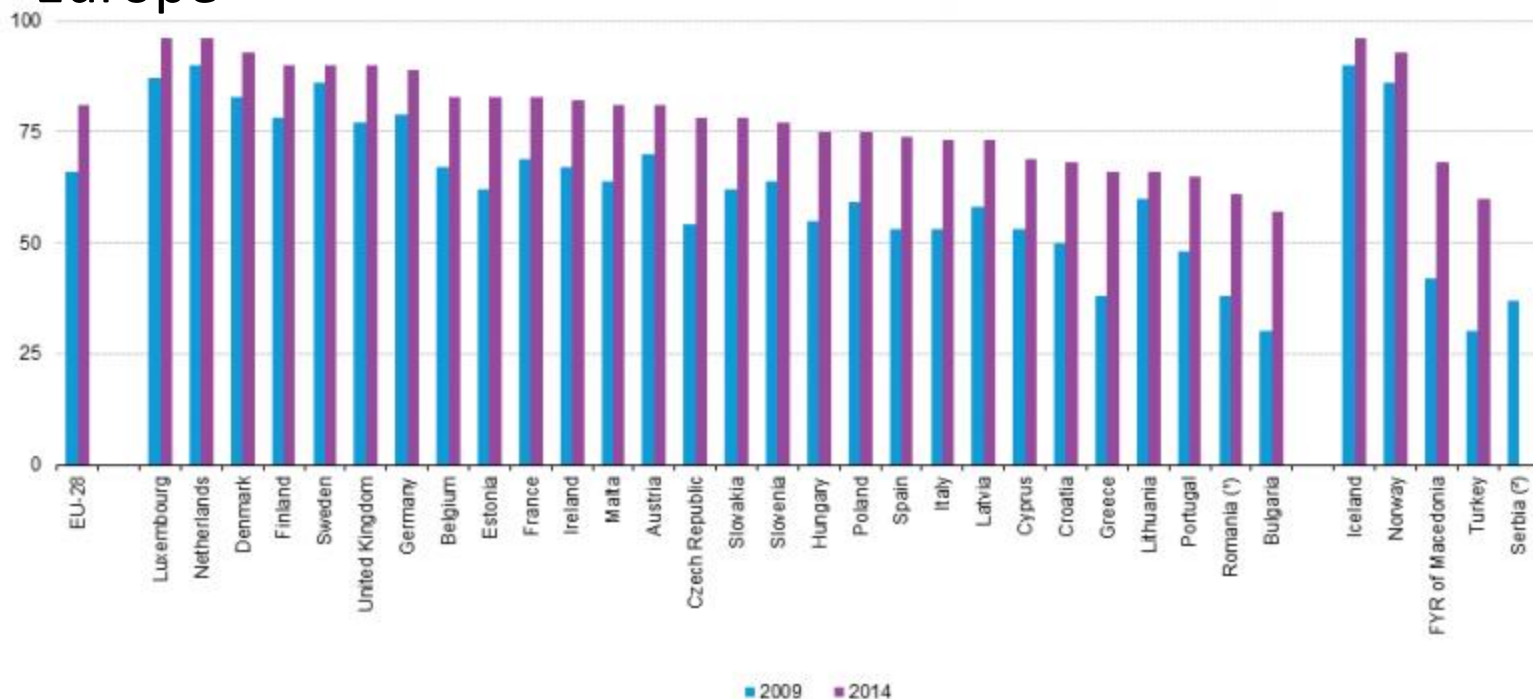
- Breast cancer patients database for research purpose



- Owned panel for a specific rare disease patient using Vision Critical tools

Sampling rare population by creating a survey panel

- Made possible by increasing internet access equipment rate in Europe



(*) Break in series.

(?) 2014: not available.

Source: Eurostat (online data code: isoc_cl_in_h)

EURORDIS survey panel



Group of pre-screened patients who have offered to provide personal information on an on-going basis



Patients, caregivers and patient representative



Contact list (email) with useful sociodemographic information (disease, gender...) combined with an emailing tool

➔ Give the possibility to select the contact you want to interrogate



Exclusive tool, constant check by data management, protected by a password with access limited to one person only

Creating a survey community : advantages for EURORDIS and its members

Gives the time to diversify your contacts

- Increases the number of diseases represented
- Increases the number of countries represented
- Increases the number of people to make valid statistical analysis

Creating a survey community : advantages for EURORDIS and its members

Gives the possibility to control the sample

- Decreases the risk of irrelevant answers (non-concerned, lobby...)
- Ensures sociodemographic balance (men/woman)

Creating a survey community : advantages for EURORDIS and its members

Expands methodological possibilities

- Interrogate sub-populations (woman with rare disease, very rare disease)
- Carry out longitudinal study

Data protection and Technical solution



The project has been approved by the CNIL (independant data protection authority in France)

Online platform with high security level

- 24 hours surveillance and access control
- Temperature maintenance
- Efficiency and Quality of AC Uninterruptible Power Systems (UPS)

Data storage in Grenoble, France : high European data protection standard

Data protected by a unique password

Work with non-profit clients : French Red cross, MSF, Grenoble university

Country coverage : a progressive approach

- The survey community will cover EU 28
- And extended Europe in the coming years




Your advocacy tool

Our objective

- Provide scientific results that will be recognized at a scientific and political level
- Reinforce rare disease community and make rare disease patients voices heard as a whole

Your advocacy tool

Scope of the surveys

- Data Sharing
 - Access to diagnosis
 - Rare disease impact on employment, revenue, schooling...
 - Access to care
 - Access to medicine
 - Off label use of medicine
-  To be confirmed and refined depending on the needs and the political agenda

Your advocacy tool

Analysis of the results

- Results by country and relevant group of countries (EU newcomers/South Europe...)
- Results by disease and relevant group of disease (policy action group/Orphanet classification)

➔ Importance of having sufficient number of people per category

What about the recruitment ?

Online survey with Sphinx

- **5 questions:** disease, gender, status (care givers, patients, patient representative), country, email
- **24 languages for the questionnaire:** Croat, Czech, Bulgarian, Danish, Dutch, English, Estonian, Finnish, French, German, Greek, Hungarian, Italian, Polish, Portuguese, Romanian, Russian, Latvian, Slovak, Lituianian, Slovak, Slovenian, Spanish, Swedish
- **7 languages for the list of disease :** based on Orphanet list

First survey : Innov care

- **End of december** : End of the RD Connect Delphi exercise
- **Begining of January** : Qualitative study about quality of life, care pathway with a focus on employment
- **Starting in May** : Quantitative study that will use the database on the impact of rare disease (on employment, schooling, quality of life...)

What does it mean to become a Patient Voices programme partner?

Involvement in the research programm

- Suggest topics that will have to be selected by the steering committee
- Give input prior to study
- Possibility to be part of the topic advisory committee
- Possibility to be involved in the analysis of the survey

What does it mean to become a Patient Voices programme partner?

- Register to the Patient Voices survey panel and participate in our surveys
- Help us find patients who want to belong to this survey panel