Sharing health data to advance scientific research and improve clinical benefits are of particular importance in the field of rare diseases where knowledge and expertise are limited and patient populations are geographically dispersed.

Understanding what patients want and need from rare disease research and data sharing is important to ensure their participation and engagement in the process, and to ensure that these wishes and needs are embedded within research and healthcare delivery design.

Within this report you will find:

1. Key results from research conducted by EURORDIS, outlining rare disease patients’ preferences with regards to data sharing

2. Seven recommendations designed to inform and support stakeholders involved in data sharing initiatives

3. Research and recommendations methodology
WHY SHARE HEALTH RELATED DATA?

Rare disease patients, regardless of the severity of their disease and their socio-demographic profile are clearly willing to share their data:

To foster research on their disease...

- 97% would be ready to share their data to better understand the mechanisms and causes of their disease
- 97% to develop new treatments for their disease
- 97% to improve diagnosis of their disease

or to improve their healthcare

- 95% to receive additional specialist advice on their care

are also willing to share their data to improve research on diseases other than theirs.

Rare disease patients seem more inclined to share their data than the general population: depending on the study, between 37% and 80% of the general population declare that they would be ready to share their health data1.

If given the opportunity, would you be willing to make your/the person you care for health information available for the purpose of:

1. The possibility to learn more information about their disease

2. The possibility to discuss and ask questions directly to professionals involved in the project

3. The possibility to be informed on the results of the project

The main incentives for patients to participate in such initiatives is the possibility to discuss and learn information about the disease and the project.

1: Attitudes towards data sharing, YouGov. 2018. https://docs.google.com/spreadsheets/d/1_-ntuc7ZExXlXvaFsfrugjNFQUPTljB677ijOv/#!edit#gid=2093652055
DATA SHARING: RISK AND LEVEL OF CONTROL

Rare disease patients want to have control over the data they are sharing.

80% said they wanted to keep full (47%) or near to full (33%) control. Being in favour of sharing data and calling for more control are not contradictory, they are clearly stated as two parallel requirements.

According to you, what are the most important risks connected with disclosure of your personal or health information?

On a scale from 1 to 5, how much control would you like to have over this information?

According to rare disease patients, the main risks associated with data sharing are:

1. **50%** Their information being shared with 3rd parties without their consent
2. **47%** Their information being used in a different context from the ones where they disclosed it
3. **35%** Their information being used without their knowledge
4. **34%** Becoming the victim of discrimination

Comparison with general population: 5% view becoming a victim of discrimination as a risk.

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### 3 HOW MUCH DO PATIENTS TRUST POTENTIAL USERS OF DATA?

Rare disease patients show a higher level of confidence in not-for-profit stakeholders.

Respondents were asked whether they trust various authorities and types of organisations to handle and use their health information carefully. Patient trust in not-for-profit stakeholders is considerably higher than trust in for-profit stakeholders.

#### NOT-FOR-PROFIT AND HEALTHCARE PROFESSIONALS

- **89%** Medical doctor involved in their healthcare
- **79%** Researcher from a non-profit organisation
- **77%** Patient organisation
- **69%** Other healthcare professionals (dentists, pharmacists, nurses)
- **48%** Government from their country

#### FOR-PROFIT

- **47%** Researcher from a private genetic testing company
- **45%** Researcher from a pharmaceutical or medical device company
- **16%** Insurance company

People involved in the project can belong to different types of organisations. How much confidence do you have in each of them to handle and use your health information carefully?

Opinions are fragmented on whether responsibility for deciding with whom information is shared, how and why, could be delegated to an ethics committee.

Only 48% of respondents reported confidence in government entities that are frequent initiators and custodians of data sharing initiatives.

Would you allow an ethics committee to decide on your behalf with whom your information will be shared, how and why?

- **49%** are in favour
- **43%** are against the idea
- **8%** don’t know
How best to communicate with rare disease patients?

Participants were asked to express their preferences regarding the types of information they would like to receive and how they would like to receive it.

The top three most important pieces of information patients would like to receive about a data sharing initiative are:

1. **80%** Details on how the project could be beneficial for their disease or other diseases
2. **51%** Brief summary of the key information necessary to understand the main aspects of the project
3. **49%** Information about the data management rules (e.g., how access to health information will be granted or is there an ethical review?)

Email and face-to-face discussion are the favoured ways of receiving information.

- **93%** Email
- **86%** Face-to-face discussion with professionals involved in the project
- **85%** A dedicated website
- **67%** Attending conferences
- **56%** A mobile app

The ideal frequency to be informed for the majority of the respondents is once a month.

- **55%** Once a month
- **21%** Once a week
- **12%** Once a year
- **6%** Several times a week
- **6%** Don’t know

How often would you like to be informed about the outcome of the project?
5 RECOMMENDATIONS FOR DATA SHARING INITIATIVES

The following recommendations have been derived from the surveys and consultations.

Policy makers should:

1. Ensure implementation of appropriate legislation at European and national levels and pursue efforts to foster cultural, technological and infrastructural changes to further develop international data sharing initiatives in health and research for rare diseases.

   Already existing initiatives in this area include:
   
   • The work undertaken by the European Reference Networks to establish a dedicated data-sharing platform enabling information exchange and mutual learning to improve rare disease patients’ diagnosis and care, while also contributing to the development of research and innovation.
   
   • The signature by several EU countries of a declaration whereby their governments commit to cooperate to deliver cross-border access to genomic information. This declaration has the potential to maximise use of health care resources and advance the development of personalised medicine especially in the rare disease field.

All stakeholders involved in data-sharing initiatives, including funders, sponsors, clinicians and researchers, healthcare authorities and professionals, patient organisations and non-profit organisations should:

2. Implement robust standards to ensure secure, ethical and responsible data sharing whilst putting in place safeguards around data protection.

   • Ethical and responsible data sharing should be enabled through widespread implementation of the IRDiRC recognised resource, the international charter of principles for sharing biospecimens and data which provides guidance for effective legally and ethically grounded data sharing.
   
   • Furthermore, several ongoing initiatives are testing the use of blockchain technology, which has proved an efficient tool to protect personal data.

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For more information on the results of the survey and the recommendations, see the article published in the Orphanet Journal of Rare Diseases:

Share and protect our health data: an evidence based approach to rare disease patients’ perspectives on data sharing and data protection or contact rare.barometer@eurordis.org.
EURORDIS-Rare Diseases Europe has organised and participated in a number of consultations with rare disease patients and carers in order to reach a comprehensive understanding of rare disease patients’ needs and expectations regarding data sharing.

Patient and carer views and perspectives were collected via the following methods:

**Multiple Approaches to Securing Patient Preferences on Data Sharing**

- **RD Connect**
  - Facilitation of the RD Connect Patient Advisory Council (PAC). RD Connect is an integrated platform connecting databases, registries, biobanks and clinical bioinformatics for rare disease research (16 members), 2012-2018

- **Rare Barometer**
  - Webinar on concerns of General Data Protection Regulation (50 participants), 2017

- **Delphi exercise**
  - Participation in focus groups on data sharing for international research (52 patients)

**A Final Large Scale Quantitative Survey**

Conducted by Rare Barometer

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**Thank you** to all people living with a rare disease who participated in the survey and discussion groups and to Rare Barometer partners!

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