

# WP8 – DELPHI EXERCISE WITH PATIENT REPRESENTATIVES



# Methodology: selection of patients

2

- Delphi exercise : consensus building method
- **15 volunteer experts patients** from 15 different countries: France, Italy; Germany; Spain; Netherlands; Canada; United Kingdom; USA; Australia; Greece; Austria; Sweden; Serbia; Switzerland; Japan
- **Diversification of the disease** represented :
  - Severity of the disease
  - Children focus organisation
  - Particular focus on kidney and neuromuscular diseases



# Methodology: questionnaire

3

- Based on the question raised during the focus groups
- Designed in close collaboration with PEALS
- Validated with PAC and PEC
- First part with open questions, second part with closed questions
- Two rounds online questionnaire disseminated through a data dissemination and analysis and survey software (Sphinx)



# Preparation of the participants

4

- Review paper presenting the RD-Connect project
- Document summarizing the main concepts related to data sharing and data protection issues in the context of RD-Connect



# Next steps

5

- Intermediate analysis
  
- 2<sup>nd</sup> round questionnaire :
  - ✓ Prioritization of the issues
  - ✓ In depth investigation of controversial issues



# First round : Preliminary results

6

- Broad consensus about the necessity of data sharing
- 15/15 respondents think that data sharing is urgently needed to accelerate treatments for rare disease patients.
- Data protection has to be ensured and purpose has to be clear
- The risk does exist but opportunities are higher than the risks
- Mainly because there is no other option for rare disease and patient believe in technological possibilities to minimize risk



# Opportunities

7

- Diagnostic and improvement of the knowlegde about the progression of the disease
- Gathering all the relevant actors around the initiative
- Make the «Governments» recognize even the most rare diseases
- Harmonisation of the rules used to collect the date



# Most important safeguards for patients

8

- Trust in the actors involved
- Patient representation in the Governance of the platform
- Information : Who get access, for what purpose, clear description of use and benefits
- The global unique identifier in order to link and harmonize the data is seen positively





# Who should participate in the Governance of the project?

9

- Need for all stakeholders to be represented :
    - Patient organisation
    - Doctors and nurse
    - Organisation of physicians, geneticist
    - Academic researcher
    - Industrie representative
    - DG research representative
    - Lawyers
- => Collective and institutional approach



# Relation to information

10

- No consensus:
  - cultural difference
  - severity of the disease
  
- Insist on the role of patient organisation, the necessity to have trainings for patient
- Insist on the fact that it is a personal choice
- They want to be informed about the incidental findings



# Example

11

- The reuse of data that were gathered for another purpose is not a problem, we should use whatever is available to advance research.

	<b>Frequencies</b>
<b>Totally agree</b>	5
<b>Fairly agree</b>	4
<b>Fairly disagree</b>	3
<b>Totally disagree</b>	3
<b>Total</b>	<b>15</b>