

Appendix 1. Text introducing the survey and questionnaire.

Thank you for agreeing to participate in this survey on a current topic with an important impact on research and health care: transparency in research and sharing and access to individual clinical trial data. Help us to know the point of view of patient and citizen associations!

Why access to individual study data is important

It is first of all a question of transparency. Access to data collected during clinical studies - done to evaluate the efficacy and safety of medical interventions, or to study the frequency of diseases or risk factors - makes clinical research "transparent". In this way, all the details of a study can be known, not just the information that are published.

Other researchers can use data already collected (made anonymous and de-identified) and respond to new research questions, in order to maximize the value of data and reduce "waste". For example, having access to the data of the studies enables to deepen the knowledge and explore the risks and benefits ratio, also for subgroups that are not of interest to those who conducted the primary study. In the case of publication in scientific journals, whoever is in charge of examining the article can check that the conclusions presented are justified by the data collected.

At this [link](#) you will find a detailed article and a box with two examples* of how individual participant data from clinical trials can be reused.

However, some issues exist. Some concern the people participating in the clinical study:

- persons participating in the studies must be informed about the possibility that other researchers can use the same data. However, it is difficult to predict who could request access to data, and for what reasons, because research questions evolve over time based on knowledge, and participants could therefore be asked to give their consent to a procedure that is not defined in detail;
- some data collected may be sensitive (for example, age, sex, hospitalization, date of diagnosis, etc.) and therefore can not be shared without appropriate strategies to avoid the identification of the person they concern. The data must in practice be rendered anonymous or, better, de-identified to minimize the possibility of tracing the identity of the participant.

Other problems concern researchers who may have planned new analyzes themselves not yet published, or fear inappropriate use or incorrect interpretation of data.

Numerous institutions - including research institutions, research funding organizations, some pharmaceutical and regulatory agencies - are in favour of sharing individual data collected during clinical trials. The scientific community is committed to defining standards and guidelines to share de-identified individual data in an effective and responsible way.

It is essential that the definition of these processes involves the people who participate in studies and patients' representatives.

This is why we ask your association to contribute to the dialogue with researchers and the scientific community.

Who we are

This survey is promoted by the Istituto di Ricerche Farmacologiche Mario Negri IRCCS. The questionnaire was prepared by Cinzia Colombo, Anna Roberto - Laboratory for citizen involvement in healthcare, and Rita Banzi - Drug Policy Laboratory of the Mario Negri Institute, Elena Parmelli - Department of epidemiology ASL Rome 1 Regione Lazio, in collaboration with Karmela Krleza-Jeric, IMPACT Observatory, Mediterranean Institute for Life Sciences, Split, Croatia.

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Data processing: information is collected at the Mario Negri Institute of Pharmacological Research IRCCS, and will be analyzed anonymously without specific reference to individual associations, and will only be used for research purposes. The results will be published in scientific and lay articles.

Questionnaire

1. Name of association _____

2. Disease of interest, field (Drop down list of options)

3. City _____

4. What is your role in the association?

- President
- Vice-president
- Secretary
- Other, please specify

5. In the past three years, has your association been involved in clinical research?

- Not at all
- Sometimes
- Several times
- I don't know

6. Please indicate in what activities the association was involved

Only those responding "sometimes" or "several times" to question 5 need reply to this question.

More than one option possible

- Setting research priorities to guide funding allocation
- Involvement in study design (for example definition of the research question, outcome selection, informed consent revisions)
- Promotion for participation in clinical studies (for example study advice on website, newsletter with trials site-posting)
- Other, please specify

7. Is your association aware about the current debate on sharing and access to individual participant data from clinical studies?

- Yes, we have discussed this in the association (for example, during meetings of the executive board or members, in public events)
- Yes, some members are involved in the discussion
- No, we are not aware of it
- I don't know

Only those responding "Yes, we have discussed this issue in the association" or "Yes, some members are involved in the discussion" continue with questions 8-13.

Those responding "No, we are not aware of it" or "I don't know" go to question 14

8. In what circumstances did the association discuss this topic? *More than one option possible*

- Encouraged by this survey
- Encouraged by members of the association (for example, a lay member of the ethics committee)
- During the definition of policies for funding research initiatives
- Encouraged by other patients' associations

- Encouraged by meetings/conferences with medical societies, researchers, physicians, etc.
- Other (for example prompted by media), please specify

9. Does your association have an official position on sharing and access to individual participant data from clinical studies?

- Yes
- No

10. How would you classify it?

Only those answering “Yes” to question 9. Only one option possible

- In favour
- Against
- In favour, with some restrictions
- Neither for nor against

11. Please, briefly explain the reasons supporting your association’s view

12. Who do you think should have access?

Only those answering “Yes” to question 7 and those answering “in favour” or “in favour with restriction” or “neither for nor against” to question 10 need reply to this question

- Only researchers presenting a clear, complete and relevant research question
- Only researchers presenting a research question that has no commercial interest
- Only researchers from public or not-for-profit institutions
- All researchers from public or private institutions (including for example from the pharma industry)
- All, not only researchers, but also persons such as representatives of patient and citizen associations, or scientific journalists with non-strictly research objectives (for example for journalistic inquiries, information on the quality of studies).
- I don’t know

13. In your opinion, which of the following aspects are important to guarantee a fair and secure process of sharing individual participant data?

Only those answering “Yes” to question 7 and those answering “in favour” or “in favour with restriction” or “neither for nor against” to question 10 need reply to this question

More than one option possible

- Data should be adequately de-identified before sharing, to reduce the risk of re-identification
- The informed consent form should explicitly report that data will be de-identified before sharing
- Anyone who decides not to share their data should maintain the right to participate in the trial (as far as possible, two separate consents)
- Trial participants should have the right to decide on any conditions under which data can be shared (for instance, only for research in some areas, only for researchers from public institutions)

- The informed consent form should explicitly specify that future research that might employ the collected data cannot be fully predicted at the time of signature of the consent
- Data should be stored in archives with high-level security standards
- Requests for access should be evaluated by the ethics committee or by an independent body
- Those who access data should sign agreements that specify sanctions in case of misuse of data or attempts at re-identification
- Requests for access should be registered and monitored and made publicly available
- The results of re-analyses should be made public
- I don't know
- Other, please specify

14. In general, what are the possible risks of sharing and access to individual participant data from clinical studies? *More than one option possible*

- Possible re-identification of participants who generated data, and privacy concerns
- Data are re-used to answer questions of limited value and quality/non-ethical research
- Data are re-used for purposes the trial participant does not agree with (for instance in other disease areas or to pursue objectives that the participant does not approve)
- The data sharing could cause unfair commercial competition that may reduce funding for research
- No risks are foreseen
- Other, please specify

15. In general, what are the possible benefits of sharing and access to individual participant data from clinical study? *More than one option possible*

- Data can be reanalyzed to answer different research questions, reducing unnecessary duplication and waste of resources
- Data can be pooled to study adverse reactions and side effects
- Data are re-used to support for drug regulatory decisions
- Results are verified by independent groups of researchers or other stakeholders
- Sharing and access to data ensures transparency
- Speeding up research progress /innovation
- Other, please specify

16. Do you think that sharing individual participant data from clinical studies would discourage people from participating in clinical studies?

- Yes
- No
- I don't know

Additional questions to describe associations and their responses better

17. How many members did your association have in 2016?

- Less than 100

- 100 to 500
- 501 to 1,000
- More than 10,000

18. Who are members of the executive board of your association? (2016 as reference year)

- All members (or the majority) are patients or citizens/lay people
- Half the members are patients or citizens
- Patients or citizens are the minority or not included
- Other, please specify

19. In the last three years, in which of the following activities has your association been involved?

Three options: yes, to some extent, no

- Education and dissemination/information
- Financial support for research
- Lobbying at the institutional level (local and central)
- Fund-raising
- Health and social support (for example home care, access to services, psychological support)
- Surveys about health services quality
- Other, please specify

20. In the last three years, which of the following financial sources have mainly supported your association's work? *More than one option possible*

- Annual membership fees, donations, bequests
- Public funding (central, regional, local)
- Funding from pharma or medical device companies
- Funding from other commercial entities
- Other sources of funding, please specify

21. Your association's work is mainly:

- Local
- Regional
- National

22. Is your association involved in European or international networks or activities?

- Yes
- No

If yes, please specify

Thank you for your cooperation

*In the article “Sharing individual participant data from clinical studies” (available at https://www.partecipasalute.it/cms_2/node/6633, accessed 21 November 2018), we presented two examples on:

- the efficacy and harms of paroxetine and imipramine in treatment of major depression in adolescence

Cited source:

Le Noury J, Nardo JM, Healy D, et al. Restoring Study 329: efficacy and harms of paroxetine and imipramine in treatment of major depression in adolescence *BMJ* 2015;351:h4320.

- the effect of dosing regimens on the antimalarial efficacy of dihydroartemisinin-piperaquine

Cited sources:

WorldWide Antimalarial Resistance Network (WWARN) DP Study Group (2013) The Effect of Dosing Regimens on the Antimalarial Efficacy of Dihydroartemisinin-Piperaquine: A Pooled Analysis of Individual Patient Data. *PLoS Med* 10: e1001564

Garner P (2013) Artemisinin Combination Therapy: A Good Antimalarial, but Is the Dose Right? *PLoS Med* 10(12): e1001565. <https://doi.org/10.1371/journal.pmed.1001565> .