

Delphi Survey on Neglected and Orphan Diseases

Project Summary

Research questions: 1) Which incentives are effective in stimulating research and development on neglected and orphan diseases?
2) Is it meaningful and/or necessary to have a regulatory instrument to frame the incentives for neglected diseases?

Research design: Delphi survey in two rounds

Survey participants: Experts and stakeholders in the fields of neglected diseases and rare/orphan diseases

Project location: Dept. of Epidemiology & International Health, School of Public Health, University of Bielefeld (Germany)

Project chairs: Professor Dr. Oliver Razum, MD, MSc
Professor Dr. Petra Thürmann, MD

Research coordinator: Angela Fehr, M.A. (USA)
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Project background / institutional affiliation

This survey is part of a research project on neglected and orphan diseases, which is conducted at the Department of Epidemiology & International Public Health at the School of Public Health, University of Bielefeld, Germany. The project is supervised by the Department Head, Prof. Dr. Oliver Razum, MD, MSc, and by Prof. Dr. Petra Thürmann, MD, Chair of Clinical Pharmacology, University of Witten/Herdecke, Philipp Klee-Institute for Clinical Pharmacology, HELIOS Klinikum Wuppertal.

Research questions

Neglected diseases and rare, or “orphan” diseases, have in common that they offer little financial incentive for investing in research and drug development (R&D). Orphan drug regulations such as the U.S. Orphan Drug Act of 1983 or the European Union’s Regulation on orphan medicinal products, enforced in 2000, were developed to stimulate orphan drug development and to ameliorate the situation for patients with

rare diseases. For neglected diseases, no such regulations exist yet. One option under discussion is to apply incentives similar to those contained in orphan drug laws to stimulate neglected disease R&D. A different approach was taken in 2005, when the draft for a Medical Research and Development Treaty (MRDT) was submitted to the World Health Organization (WHO), designed to “create a new global framework for supporting medical research and development.”

With this research project, we are asking experts and stakeholders to share their opinion on the necessity of a regulatory instrument to stimulate neglected disease R&D, and on the measures such regulation would have to include.

Method

The method chosen for this research is a **Delphi survey**. Delphi surveys are anonymous surveys among a heterogeneous group of experts who represent various perspectives to an issue under research. Owing to rankings and priority-settings, the method and its outcomes are credible and operational for policy-makers.

In this survey, you are asked to give priority judgment on causes for the problem of neglected diseases, as well as on possible solutions. The questionnaire we have developed covers a number of causes as well as measures included in the U.S. and the European regulations on orphan drugs ¹. Additionally, we have listed approaches from the draft Medical Research and Development Treaty ².

Delphi surveys are conducted in several rounds. **This survey has two rounds**. In the first round, you are asked to rank the items given in the questionnaire, and, if you wish, complement them with your own suggestions. The questionnaire for the second round (in which only first-round participants can participate), will include these additions made by participants. Also in the second round questionnaire, you will see frequency distributions of the answers given in the first round. If you wish, you may change your priority judgment based on these results.

¹ <http://www.fda.gov/orphan/oda.htm>;
http://eur-lex.europa.eu/smartapi/cgi/sga_doc?smartapi!celexapi!prod!CELEXnumdoc&lg=en&numdoc=32000R0141&mode=l=guichett

² <http://www.cptech.org/workingdrafts/rndtreaty4.pdf>).

Final frequency distributions from the second round will be mailed to you after the survey has been closed and evaluated.

Technical Procedure

The Email you received contains the link to the questionnaire for the first round. Participants in a pre-test needed around **10 minutes to complete** the questionnaire. The first round can be accessed for two weeks starting today.

Participants

We are contacting about 400 experts and stakeholders worldwide from the field of neglected and rare diseases, who took part in international conferences on the issues of neglected and rare diseases and/or are authors of relevant scientific publications on the topic. Their professional affiliations are in academia, industry, international organizations, national governments/parliaments or non-governmental organizations.

The survey is anonymous. Participants' identities will not at any time during the analysis and publication of data, be visible to other survey participants or correlated with individual survey results.

Goal

The goal of this survey is to learn which R&D-stimulating measures are given preference by the participating experts and stakeholders.

We are very grateful for your support of this research project. Please do not hesitate to contact us if you have any questions about this survey.

*On behalf of the project team,
Sincerely,
Angela Fehr, Research Coordinator*

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