orphanet

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Procedural document: Data collection and registration of patient organisations, umbrella organisations and alliances in Orphanet

https://www.orpha.net/ https://www.orphadata.com/

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I. Introduction

1. Purpose/objectives

For patients affected by a rare disease and their families, support and information are vital ressources. It is equally important for them to be able to share their experiences and connect to the rare disease community.

Orphanet offers, amongst a range of expert resources on rare diseases, a directory of patient organisations, umbrella organisations and alliances to help in this process.

This document aims to define the set of criteria used to select, register and update patient organisations and alliances.

2. Disclaimer

The content of this procedural document represents the views of the author only and is his/her sole responsibility; it cannot be considered to reflect the views of the European Commission and/or the Consumers, Health, Agriculture and Food Executive Agency or any other body of the European Union. The European Commission and the Agency do not accept any responsibility for use that may be made of the information it contains.

3. Range of application

The present procedure applies to all patient organisations, umbrella organisations, and alliances of patient organisations registered in Orphanet. The registration and update of this data is performed by the Orphanet national teams (ONT) having signed a Network Agreement (NA) and a Data Transfer Agreement (DTA) with the Orphanet Coordinating Team (OCT).

4. References

- Orphanet Standard Operating Procedures
- Regulation (EC) N°141/2000 of the European Parliament and of the council of 16 December 1999 on orphan medicinal products

5. Definitions

Alliance of patient organisations: Structure linking regional, national, European or international support groups, providing support for all rare diseases.

Data transfer agreement (DTA): Contract between the providing and recipient institutions that governs the legal obligations and restrictions, as well as compliance with applicable laws and regulations, related to the transfer of such data between the parties.

European Reference Networks (ERNs): Virtual networks involving healthcare providers across Europe, aiming to facilitate discussion on complex or rare diseases and conditions that require highly specialised treatment, and concentrated knowledge and resources.

EURORDIS: Non-profit alliance of patient organisations representing over 1000 rare disease patient organisations in 74 countries that work together to improve the lives of people living with a rare disease in Europe.

Expert resources: Experts services comprise expert centres and networks of centres of expertise, medical laboratories and diagnostic tests, patient organisations and umbrella organisations, patient registries, mutation databases, biobanks, research projects, clinical trials and platforms of interest for rare diseases in the countries of the Orphanet Network.

Information scientist (IS): Member of an Orphanet National Team, responsible for the collection, assessment, and registration of data.

Orphadata: Platform developed by Orphanet to provide the scientific community with comprehensive, high-quality and freely accessible datasets related to rare diseases and orphan drugs, in a reusable format (https://www.orphadata.com/)

Orphanet coordinating team (OCT): French US14 Inserm-based team coordinating the Orphanet Network. It produces the English Orphanet Nomenclature and is responsible for coordination of the production of the scientific content and of all Network activities including translation.

Orphanet national teams (ONT): Teams located in each participating country of the Orphanet network, and endorsed by national authorities. An ONT is composed, at least, of a country Coordinator who is responsible for the national Orphanet activities including translation. It can also include one or several information scientists, translation staff and a project manager.

Orphanet national websites: Each Orphanet national team maintains a national entry point to Orphanet, providing information on the team and on the latest news and updates concerning national activities, in the national language of the country concerned.

Orphanet Network: Orphanet national teams contributing to the Orphanet database in the framework of the signed Network Agreement and DTAs.

Patient organisation: Non-profit organisation or foundation, providing support and/or information to patients suffering from a rare disease.

Rare disease (RD): a disease that affects less than five in 10,000 persons in Europe, as defined by the European Regulation on orphan medicinal products (Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products).

Rare Diseases International (RDI): Global alliance of Persons Living with a Rare Disease (PLWRD) of all nationalities across all rare diseases

Service contract: Contract, established by Inserm-Transfert, allowing for-profit companies/organisations to access Orphanet data via a for-fee Orphadata account. The contract stipulates the conditions of use of the data.

Umbrella organisations: Structure regional, national, European or international support groups, specific for one rare disease or group of rare diseases.

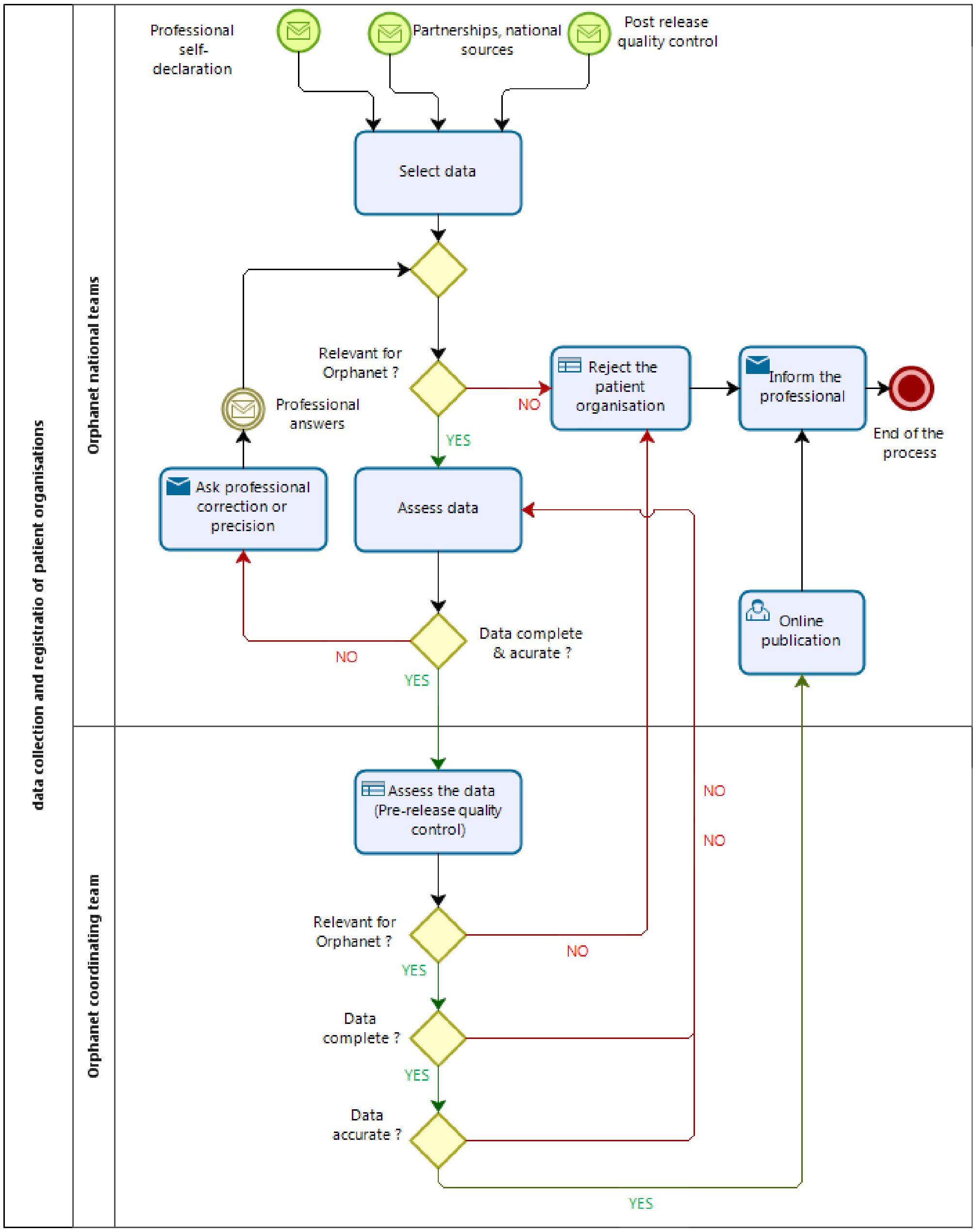
6. Filing and updates

This document is updated by the Orphanet coordinating team (OCT) as often as necessary and at least once a year. The most up-to-date version is available on the Orphanet website:
https://www.orpha.net/orphacom/cahiers/docs/GB/patient_org_R2_PatCar_PO_EP_09.pdf

II. Methodology

1. Flowchart

The general process for data collection, registration, validation, and quality control concerning patient organisations, umbrella organisations, and alliances of patient organisations is presented below:



[1]

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[1] Powered by Bizagi

2. Description

The process of registration/update of patient organisations, umbrella organisations and alliances of patient organisations starts with:

- Professionals declaring their activity in any communication channel with Orphanet teams (e-mail, phone call, etc.).
- An exchange of data through a partnership with a source of data (e.g. national alliances).
- A post-release quality control tasks focused on patient organisations, umbrella organisations or alliances of patient organisations

An annual update is organised and launched by the Coordinating Team (OCT) and National teams. All the heads and contact persons of patient organisations / alliances registered in the database are invited to review and update their activities. National teams are responsible for the follow-up of their feedback.

3. Sources of information

Orphanet national teams (ONT) are in charge of identifying the sources of information for patient organisations and alliances in their country, and are advised to establish partnerships with them to be as **exhaustive** as possible.

In case of establishing a partnership, national teams must inform the coordinating team, as some types of partnerships require the signature of a data transfer agreement (DTA).

<u>The main sources of information are:</u>

- National and regional alliances of patient organisations,
- EURORDIS,
- European Reference Networks (ERN)
- Rare Diseases International (RDI)
- Physicians and researchers working in close collaboration with these support groups,
- Congress, symposiums, forums,
- A patient organisation or an alliance declaring its activity in any communication channel with Orphanet teams (e-mail, phone call, etc.)..

4. Data selection

ONT are involved in collecting and registering information on patient organisations and alliances located in their own country. National teams and the coordinating team are involved in collecting information on European and international alliances, but only the coordinating team can register them.

National teams start the data selection by verifying that the patient organisation meets the inclusion criteria for Orphanet.

a) Inclusion criteria for patient organisations, umbrella organisations and alliances:

In order to be registered in Orphanet, a patient organisation / alliance:
- has to provide support for patients living with a rare disease, a group of rare diseases or rare forms of common diseases.
- has to be part of Orphanet Network,
- has to be responsive and must be able to be contacted by telephone, e-mail, etc.
- should have a designated head and/or a contact person.
- if the organisation has a website, the information on the disease(s) must be freely available for all users, and should not contain advertising for treatments.

The regional patient organisations are accepted if there is no national equivalent.

For an organisation with official contact points in several countries: the organisation must be registered in the country where it has its legal status, and all the official contact points have to be included as well.

b) Exclusion criteria for patient organisations:

Orphanet does not register a patient organisation / alliance if it is:
- a patient organisation dealing with non-rare diseases.
- a fund-raising trust or foundation that helps one or several patients with no real advice or help given to others
- a learned society
- a research-funding trust/foundation
- a blog or/and forum only

c) Exclusion criteria for umbrella organisations and alliances

Orphanet does not register an umbrella organisation or alliance, if the resource is:
- a group of regional patient organisations[2].
- a patient organisation with several contact points in the same country with different languages[3]
- a patient organisation in a given country with contact points in others.

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[2] However, the corresponding national patient organisation will be registered in Orphanet if it complies with the Orphanet inclusion criteria

[3] As they are also registered in Orphanet as national patient organisations with contact points

d) Criteria for countries not belonging to the Orphanet Network

Patient organisations and alliances in countries not belonging to the Orphanet network can be registered in the database by the coordinating team only if:

- they are an alliance covering all the rare diseases and/or
- are part of EURORDIS and/or Rare Diseases International (RDI).

5. Data assessment

If the patient organisation / alliance complies with the Orphanet inclusion criteria, the national IS analyse the information to check that the mandatory dataset (cf below) is provided and that it is coherent, and eventually introduce the necessary corrections before submitting to pre-release quality control.

Orphanet dataset for patient organisations, umbrella organisations and alliances

Mandatory and optional datasets are the same for patient organisations and alliances:

i. Mandatory dataset

- Name of the patient organisation in local language
- Country and city where the headquarters of the association is located
- Name and contact details (email address and/or phone number) of the head of organisation and/or contact person (for patient organisations)
- Name and contact details (email address and/or phone number) of the coordinator of the alliance (for alliances)
- The disease(s) or group of diseases covered by the organisation
- The geographical coverage of the organisation
- Membership in a European Reference Nework (ERN)

ii. Optional dataset

- Website
- Name of the patient organisation in English

6. Pre-release quality control (PrRQC)

Once the patient organisation, umbrella organisation or alliance passed the national assessment phase, the OCT performs a pre-release quality control to assess the relevance and correctness of data collected by the national teams.

This quality control is mainly focused on the disease(s) linked to the organisation as well as on the coherence of the whole dataset.

In case that some information is missing or needs correction, the form is sent back to the national teams who resubmit the data once the corrections are done.

7. Data publication

Once the pre-release quality control step has been completed, the information on patient organisations and alliances is accessible on the Orphanet website and can be retrieved from Orphadata after signing a Data Transfer Agreement (DTA) (for academia) or a service contract (for private companies). Once published, the ONT are in charge of informing the professional(s) that the activity has been published.

8. Post-release quality control

The post-release quality control for patient organisations includes the quality control projects, which are organised by the OCT on a regular basis to check the completeness and consistency of the data. ONT can, at any time, perform an extraction from the database to perform this post release quality control.

For any questions or comments, please contact us: contact.orphanet@inserm.fr

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