PatientPartner

Table of contents

About this guide ..............................1
Who wrote this guide? .................1
Who is this guide for? ..................2
What is in this guide? ..................2
A note on language ..................2
Clinical Trials ................................3
What is a clinical trial? .................3
How are they run? ....................3
  Phase I ..................................5
  Phase II ..................................5
  Phase III ..................................5
  Phase IV ..................................6
How to get actively involved ..........7
Benefits of Patient Involvement ......7
Types of patient involvement ......7
  Driving Force .........................8
  Co-researcher ......................9
Reviewer ..............................9
Advisor ............................10
Information Provider .............10
Research Subject ..................10
Stages of Patient Involvement ....13
  Pre-Approval .........................13
  Design ................................13
  Recruitment ..........................13
  Dissemination ......................14
  How to find Clinical Trials ....15
Who to get involved with ............17
  The Pharmaceutical Industry ....17
  Academics ..........................20
Ethical Principles of Partnership ...23
  Mutual Respect ...................23
  Trust .................................23
  Integrity and Credibility .......23
  Reliability ..........................24
  Accountability ....................24
  Acknowledgement .................24
  Transparency .......................25
  Sustainability ......................25
Other Clinical Research ............27
  Biobanking .........................27
    What is a Biobank? .............27
    How to join a Biobank .........27
  Registries ..........................28
    What is a registry? .............28
    How is it different from a
    Biobank? .........................28
  Paediatric Research ............29
    What is Paediatric Research? ..29
    How to get involved in
    Paediatric Research ............29
Glossary ..........................31
Useful Resources ....................33
Acknowledgements .................37
About this guide

Who wrote this guide?
This guide was produced by PatientPartner, a three year project funded by 7th Framework programme of the European Commission.

For more information on the 7th Framework programme please visit the European Commission website: cordis.europa.eu/fp7/understand_en.html.

There were four partners working on the PatientPartner project: the European Forum for Good Clinical Practice (EFGCP), the European Genetic Alliances Network (EGAN), Genetic Alliance UK and the Dutch Genetic Alliance (VSOP).

The PatientPartner project aims to identify patient needs for partnership in the clinical trial process. The project firstly examined the existing views, needs, practices and experiences of patients. This involved literature reviews, interviews with Patient Organisations, opinion leaders and other clinical trial stakeholders as well as a European survey on patient involvement in clinical trials to identify good practices.

PatientPartner also conducted a series of workshops to promote the dialogue between Patient Organisations, pharmaceutical companies and researchers on patient involvement in the clinical trials’ context.

Who is this guide for?
This guide has been created for Patient Organisations and patient representatives. It provides them with information and tools necessary to open communication between themselves and other stakeholders involved in clinical research, by providing information on the clinical trial process.
If you are a sponsor or investigator, please refer to our other guide: “Patient Involvement in Clinical Research: A guide for Sponsors and Investigators” which can be downloaded from our website: www.patientpartner-europe.eu.

What is in this guide?
This guide contains information on ways in which Patient Organisations and patient representatives can develop better and more open communication with other stakeholders involved in clinical research. The suggestions that are made have been derived from background reading, surveys, interviews and workshops organised and conducted by PatientPartner.

All of the background information used in this guide can be accessed from the PatientPartner website: www.patientpartner-europe.eu.

A note on language
Words or terms which have been written in italic are defined in the “Glossary” section at the end of this guide.

Organisation names and Acronyms written in bold are detailed in the “Useful Resources” section at the end of this guide.

As PatientPartner is a Europe-wide project, we appreciate that English may not necessarily be your first language and have attempted to employ commonly used terms.
Clinical Trials

This chapter on clinical trials aims to give you general information, for more details please refer to EGAN’s “FAQ on Clinical Trials” which is available in English, German, French, Bulgarian and Greek from their biomedinvo4all website: www.biomedinvo4all.com/en/publications/faq-on-clinical-trials-various-languages.

What is a clinical trial?
Clinical trials are research studies involving people (healthy volunteers or patients) that test the safety and effectiveness of a new treatment. This can also sometimes mean comparing whether a new treatment is better than existing alternatives. No matter how promising a new treatment may appear during laboratory tests, clinical trials are necessary to identify benefits and risks.

Clinical trials are designed by groups of medical and other specialists. The trial design is usually based on a thorough analysis of existing research, and a realisation that certain questions about treatment or symptom control need to be answered. It is discussed with medical staff, nurses, patients, statistical experts and support staff, as well as representatives from drug companies, to draw up the best possible trial design. The design for the study is known as the “protocol”.

How are they run?
In order to obtain approval for a clinical trial, the study protocol must be submitted to health authorities and reviewed by an Ethics Committee which ensures that the research respects the dignity, rights, safety and well-being of the people who are taking part. In order to ensure compliance with ethical standards, the majority of clinical trial protocols are developed in line with the “Declaration of Helsinki”, a set of ethical standards for research involving human beings, human material or identifiable data, devised by the World Medical Association.
Phase I
- Establish safe/tolerable levels
- Establish initial pharmacology in humans
- Usually carried out on healthy volunteers

Phase II
- Provide initial evidence of treatment effect and gather more safety data
- Compare effects with placebo
- Define dosage and treatment schedule
- Includes participants with disease

Phase III
- Assess risks and benefits
- Compare benefits/side effects with other drugs or placebo
- Includes participants with disease

Phase IV
- Post Marketing Surveillance Stage
- Once the drug or treatment has been approved
- Evaluate long term effects and less common side effects

N.B.: Clinical trials can sometimes include more steps such as splitting Phase II into: Phase IIa and Phase IIb

For more information on how clinical trial protocols are reviewed in your country, go to the European Forum for Good Clinical Practice (EFGCP) website: www.efgcp.be/EFGCPReports.asp?L1=5&L2=1.

Clinical trials can be led by different stakeholders; however, the basic structure in which the trials take place remains the same with four phases each representing different stages.
Phase I
Phase I trials, sometimes called early treatment trials, aim to test the safety and pharmacology of various doses of a new drug. Pharmacology means looking at the effects of a drug on the body which includes looking at possible side effects (e.g. does it cause depression, raise blood pressure, etc.). Phase I trials involve only a small number of participants who are usually healthy volunteers. In exceptional cases, for instance with cancer, HIV or gene therapy trials; patients who are at a very advanced stage of the disease may participate.

Phase II
Phase II of a clinical trial tests the new drug or treatment in a larger group of people who are ill, to see whether it has any effects suggesting that it might help them. As in Phase I, the number of participants is limited. Phase II trials also look at safety and dosage and compare effects with placebos.

Phase III
Treatments only move into a Phase III clinical trial if Phases I and II suggest that the new drug or treatment might actually be useful in ways that patients would regard as important.

Phase III trials test new drugs or treatments in larger groups of people who are ill. Phase III trials compare the new drugs with whatever treatments are currently in use, or occasionally with a placebo. These trials look at how well the new treatment works in practice, and any side effects. They usually last longer than Phase II trials – typically a year or more.

Often several thousand patients in different countries will be involved in a Phase III trial. A large amount of participants is necessary because investigators have to be able to detect moderate but important differences between treatments. However in the case of rare diseases this is not
possible therefore Phase III trials are sometimes conducted with a smaller number of patients.

Phase IV
Also known as the post-marketing surveillance stage, and occurs once the new treatment or drug has been approved.

After a medicine has been launched, Health Authorities often ask companies to collect additional data from the actual use of the medicine in thousands of patients. Phase IV studies are designed to provide broader experience in evaluating the safety and effectiveness of the new medicine in larger subgroups of patients, and to compare and/or combine it with other available treatments.

These studies are designed to evaluate the long term effects of the drug. Less common side effects may also be detected at this stage.

Despite the vigilant tests carried out during clinical trials in the other three Phases, active post-marketing surveillance of drug side effects is essential. Not all potential side effects of a medicine can be anticipated based on only several hundred to several thousand patients. Therefore, clinical trial sponsoring enterprises maintain a system of risk assessment programs to identify side effects that did not appear during the other trials. Information on adverse events that have been brought to the attention of the marketing authorisation holder, are collated. This is also called “Pharmacovigilance” (i.e. safety of medicines).
How to get actively involved

Benefits of Patient Involvement

Patients and Patient Organisations have much to contribute to scientific research. Their involvement within the research process is vital.

Patients are able to offer a unique perspective based on their *experiential knowledge* (i.e. knowledge acquired through dealing with the effects of their condition on a daily basis). In the pre-approval and design stages of research, the input of patients enables the development of more efficient trials that address issues expressed by those living with the condition.

Active involvement in clinical trials can also mean that patients have access to more information on the latest treatments and technologies and may be able to access new drugs and treatments before these become available to others. By helping with recruitment and dissemination of the trial results, Patient Organisations are not only supporting research but ensuring that information is presented in a patient friendly way.

Types of patient involvement

This diagram of patient involvement in clinical research represents the different levels in which Patient Organisations and patient representatives can get involved in the clinical trial process: As a driving force, a co-researcher, a reviewer, an advisor, an information provider, and as a research subject. Originally the six levels were put on a vertical ladder, but time and research has confirmed that they should not be viewed in a hierarchy but rather horizontally since all roles are of equal importance.
Driving Force

The term Driving Force is used to describe a person or group of people who start a process and keep it going. By being the Driving Force behind a clinical trial, a person or group representative can actively promote and push for the running of a clinical trial or development of a new treatment. In clinical trials, the Driving Force could also lobby for certain research to be performed, finance the trial, gather researchers to perform it, etc.

The AFRT (French association for research on Trisomy 21) is devoted to promoting research in Down’s Syndrome (Trisomy 21) and does so by giving grants and fellowships and publishing issues of “Nouvelles du Chromosome 21” (News on Chromosome 21) which provides information on medical, scientific and social events concerning Trisomy 21.

Created in 1990, the AFRT is run solely by parents whose children are affected by Down’s Syndrome with only a few of the board members being medical professionals. In the last 12 years, the AFRT have contributed over €370 000 to fund research. Some of their most recent projects include a “Molecular analysis of cognitive and behavioural deficits in Trisomy 21” and research into the “Role of the autonomic nervous system in the onset of fatigue during exercise in young adults with Trisomy 21”.

For 2010-2011, four grants were awarded to Bachelors’ and Masters’ research projects. A grant was also awarded to fund the realisation of a DVD to accompany research previously supported by the AFRT on speech therapy.
Although (co) funding trials is a great way to promote research, Patient Organisations can also push for research by gathering researchers and demonstrating interest in new treatments (see case study in “Academics”, p.21) Patient Organisations wanting to develop research protocols should bear in mind that there are strict regulations and should therefore check requirements for ethical approval. The European Forum for Good Clinical Practice (EFGCP) provides reports on the ethical review of protocols for most of the European countries and also provides an index of the relevant websites: [www.efgcp.be/EFGCPReports.asp?L1=5&L2=1](http://www.efgcp.be/EFGCPReports.asp?L1=5&L2=1).

**Co-researcher**

The term co-researcher is used to describe a person who is actively involved in research, to the extent that they are seen by their "professional" colleagues as a partner rather than someone who might be consulted occasionally.

There are a variety of ways in which Patient Organisations and patient representatives can be actively involved. For example, they can help translate research results into a patient friendly format, co-write a scientific article, gather research data and information or even lead focus groups or discussion sessions as part of the research.

**Reviewer**

A Reviewer is a person who is asked to give his or her opinion on a written document. He or she can suggest changes according to his or her group's point of view.

In clinical trials, one could be asked to review e.g. a clinical trial protocol, a patient information sheet, an informed consent form, a grant proposal. Reviewers will usually be selected on the basis that they are able to act as representatives of patients with a certain condition.
Advisor
An advisor is a person or representative of a group who gives advice to another party. In clinical trials, a person or group representative could be asked to advise on explaining informed consent to patients, how to recruit patients to a trial, which topics merit research, etc.

A patient representative may be asked to advise a clinical research program committee on the development of a trial. They may be asked to advise a European regulatory authority committee or ethics committee.

Information Provider
An Information Provider is a person or group representative who provides information to another party. In clinical trials, this can be on how and where to participate in the clinical trial, the informed consent form, experiences of having a medical condition etc.

Sponsors and Investigators sometimes turn to information providers to supply them with demographic and/or other characteristic information on the members that are represented by the Patient Organisation. They may also be consulted for disease specific information to be used in a clinical trial.

Research Subject
Research Subject is the term used for a person who is involved in a research experiment as a participant. In a clinical trial this could involve testing a new drug or treatment or contributing DNA, blood samples etc. For Patient Organisations, involvement can mean helping with recruitment of research subjects, informing their members about research opportunities and facilitating communication between all the stakeholders involved in the research process.
Shirley was invited to be a research subject in a dental clinical trial after being referred to her local dental hospital. Over a period of several weeks she was asked to trial three different types of dentures, fill in questionnaires about their comfort and use, and attend an appointment once a week to check on her progress. During the course of her trial, she received excellent care and was glad to be contributing to the research. Once the trial was over, Shirley was approached to join a clinical management team in which she would help review and comment on new trials. “I found it fascinating” said Shirley, referring to the experience of being part of the clinical management team. Not only was her opinion valued and taken into account, she felt as though she was given ample opportunity to raise questions and express her opinion about the trials. Her unique perspective as a patient with experience in wearing dentures allowed her to make valuable amendments to trial protocols. For example, she pointed out that quality of life is an important issue and that patients with dentures can sometimes feel self-conscious about wearing false teeth. She also referred back to her own experience in the trial in which she was asked to fill in a questionnaire, pointing out that this did not leave much scope for commentaries. She therefore suggested that the new trial require participants to keep a diary, allowing investigators the opportunity to gain a more in-depth understanding of possible issues which they might not have previously considered.

For more information please see Dr Sue Pavitt’s article in the INVOLVE newsletter entitled: “The impact of PPI on clinical trial design and operations – can we demonstrate value for money?”. (www.invo.org.uk/pdfs/Autumn2010d2.pdf)
Patient Organisations and patient representatives can be involved in several stages of research. Their input is vital in ensuring that the research addresses the appropriate issues and provides suggestions for cost effectiveness.

**Pre-Approval**
It is useful to have the input of Patient Organisations at the pre-approval stage as they can comment on whether the disease condition they represent would truly benefit from the research. They can also comment on the potential size of the recruitment pool and whether there is demand for new/better treatment.

**Design**
Patient representatives can comment on operational factors which will help determine whether the timescale of a clinical trial is realistic. Case studies have demonstrated that taking into consideration participant’s constraints ensures better recruitment and compliance. From a participant’s point of view, this should also ensure that taking part in the trial can be fit around their everyday obligations.

At this stage, Patient Organisations and patient representatives can also comment on any material and information which will be used to ensure clarity of language.

**Recruitment**
Patient Organisations can play a vital role in helping with recruitment. By forwarding the information regarding trials onto their members, they allow potential participants the opportunity to know about research that is taking place.

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### Stages of Patient Involvement

- **Choosing the type of patient involvement**
- **Choosing the stage of patient involvement**
- **Initiating contact and partnership**
- **Conducting research**
- **Dissemination and Feedback**
Stages of Patient Involvement

There are several stages in which Patient Organisations and patient representatives can be involved. Their input can be vital in ensuring that the research addresses the appropriate issues and may provide suggestions for cost effectiveness.

Pre-Approval
It is useful to have the input of Patient Organisations at the pre-approval stage as they will be able to comment on whether the disease condition they represent would truly benefit from the research going forward. They can comment on the potential size of the recruitment pool and whether there is demand for new/better treatment.

Design
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Recruitment
Patient Organisations can play a vital role in helping with recruitment. By forwarding the information regarding trials onto their members, they allow potential participants the opportunity to know about research that is taking place.
When searching for clinical trials, either nationally or internationally, you should always look in more than one place and ask for several opinions. Your doctor or a clinic you visit might know of a trial that is taking place. It is always preferable to ask your primary care doctor for their advice however you must bear in mind that they may not be aware of certain trials taking place and may therefore be unable to provide you with any more information.

Clinics usually provide information about clinical trials through posters and flyers they display in their buildings. It is also likely that the medical support group of a Patient Organisation would know where to look or who to get in contact with.

A lot of information can be found online, but be aware that not everything found on the internet is reliable.

Most pharmaceutical companies will have information about the clinical trials they are funding, or ones that have finished on their own websites. There are also websites that have been created to help you find clinical trials such as clinicaltrials.gov, a website from US National Institutes of Health which advertises studies in the USA and abroad, and the EU clinical trials register website www.clinicaltrialsregister.eu, which contains information on interventional clinical trials on medicines where the investigator sites are in European Union member states and the European Economic Area.

Orphanet which provides information on rare diseases also has a list of research and trials occurring in several countries, the information is available in French, English, Italian, Spanish and German www.orpha.net.

Dissemination
Patient Organisations can help keep patients informed about research and results by helping with dissemination. This ensures that the information reaches more than just the scientific community.
How to find Clinical Trials

When searching for clinical trials, either nationally or internationally, you should always look in more than one place and ask for several opinions. Your doctor or a clinic you visit might know of a trial that is taking place. It is always preferable to ask your primary care doctor for their advice however you must bear in mind that they may not be aware of certain trials taking place and may therefore be unable to provide you with any more information.

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Some countries have national clinical trial sites, for example the site www.notre-recherche-clinique.fr provides the French public with information on clinical trials, what they are and how to participate in research taking place in France and the French overseas departments and territories.

It may also be worth finding out whether a disease specific research network has been set up in your country. In the UK for example, the National Cancer Research Network (NCRN) was set up in 2001 to support clinical research studies and is able to provide information on trials and how to get involved: www.ncrn.org.uk.

If you are looking to participate in roles other than that of Research Subject (i.e. as a driving force, co-researcher, etc.) it is preferable to start by approaching a clinic, pharmaceutical company or academic, as trials usually only appear on trial finder websites for recruitment purposes (i.e. to search for research subjects).
Who to get involved with

The Pharmaceutical Industry

Pharmaceutical companies come in all different shapes and sizes. There are small national companies, as well as multi-national companies. It would be best to find some background information on the companies before getting in touch with them or starting involvement.

It may be easier to work with smaller and local pharmaceutical companies as they are closer and tend to run fewer trials at the same time. Information on the company’s history may be harder to find as they may not have been involved in as many trials, however they may be more willing to share information.

Finding and choosing a pharmaceutical company to work with can be intimidating. Before looking for a pharmaceutical company, you first have to decide what type of involvement you would like to have in the trial. Would you like to be involved in the development of the research protocol? Would you like to be involved in Recruitment or as Patient Representatives on a committee? Or perhaps you would like to help with the dissemination of information and results by putting articles in your website informing patients (see the chapter “How to get actively involved” for more information).

Here are a few questions to ask pharmaceutical companies that may be helpful in deciding whether working with them would suit you:

- How long has this company been in operation for?
- Have you worked with Patient Organisations before? And if so, in what ways?
- What would you do if a trial you were leading went wrong?
- Where do you publish your results?
- Does the company have a code of practice for working with patients and Patient Organisations? (for example the EFPIA code of practice on the relationship between the pharmaceutical industry and Patient Organisations)
In order to find which trials pharmaceutical companies are working on you can go to the website of the company of interest. Most if not all websites have links to their own trial sites where information can be found on both past and current trials. If you are not aware of particular companies working in your condition area, the European Federation of Pharmaceutical Industry and Associations (EFPIA) which represents the pharmaceutical industry operating in Europe, has a list of its members available on their website (www.efpia.org). General trial sites such as clinicaltrials.gov or www.clinicaltrialsregister.eu also provide information on the pharmaceutical company leading the trials.

However, it is important to know that not all trials are lead by pharmaceutical companies, especially in the case of smaller trials.
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GlaxoSmithKline (GSK) and a roundtable of cancer service users

In July 2010, GlaxoSmithKline facilitated a workshop for 30 cancer service users actively involved in clinical research to brainstorm ways in which to improve the design and recruitment into trials, building a user-led research process in the NHS (the National Health Service in the UK.) This was really successful and everyone who took part was keen to meet again.

Consequently, 10 service users were invited to a roundtable discussion in December 2010 to discuss the role of information and how informed choice for patients about research options could be offered alongside their informed choice about treatment options. A report co-produced by the service users was written and presented to the Under Secretary of State for Health, Earl Howe who invited its authors for a meeting to discuss possible improvements. This successful partnership is a good example of how patients and industry can come together to contribute to policy development and make a difference in how health services are run in their country.

The full report can be accessed on the NCRN (National Cancer Research Network) website (www.ncrn.org.uk) in the Consumer Liaison Group section, under Patient & Public involvement.
Academics

Academic clinical trials tend to be carried out at local or national level and often take place in hospitals. An efficient way to find and choose academic researchers to work with would be to contact the Patient Organisation’s Medical Advisory Board as they may be able to help you get in touch with researchers. In some cases, health professionals involved with patient support groups may also have a research interest in the condition they work with.

Another way would be to go through universities and hospitals which tend to provide information on up and coming clinical trials or trials that they are taking part in.

Once you have found a researcher you would like to work with it is recommended that you meet with them in person in order to discuss aspects of the trial. You may wish to see a copy of the research proposal or protocol.

Here are a few questions that might also be worth asking when getting in touch with an academic researcher:

- How long will the trial last?
- Who’s carrying out the research?
- Have you conducted clinical trials before?
- Who’s sponsoring the research?
- How will I be kept informed on the progress of the research?
Hanna Milczarek from the Dina Radziwillowa Children Heart’s Foundation: (sercedziecka.org.pl)

“The genesis of the co-operation between our Foundation and Prof. Kohl from the University Clinic Giessen-Marburg in Germany was initiated last year, prior to a conference we were organizing in Lodz in Poland. The subject of the conference was the 20th anniversary of the paediatric cardiology and cardiac surgery of the Polish Mother’s Hospital Research Institute in Lodz combined with a working session for the parents of the CHD children. I invited Prof. Kohl for a presentation of his unique prenatal surgery methods, especially regarding the hearts of foetuses. Prof. Kohl was so kind to share with me his idea and the very first results of his genuine research regarding the Materno-Fetal Hyperoxygenation Method (MFHO) and I decided to further investigate if we could conduct such a research in Poland according to Prof. Kohl’s protocol.”

The project is still in early stages however the foundation is playing an active part in its development as a driving force, establishing strong partnership with academics. They have invited five leading Polish prenatal cardiologists and ultrasound specialists to the project who have committed their participation and assuming the trial will be conducted as planned, are organising a one-day conference on the method and the newest results in Warsaw on the 10th of September 2011. Due to the fact that there are presently no EU funding programs compatible with the trial, the foundation is finalizing the budget of the trial and acquiring financing for it including sponsorship from private companies. The foundation has also taken on the role of facilitating recruitment by informing potential participants and ensuring that pre-selection of eligible patients is done by specialists in health centres. Finally information and results of the trial will be disseminated through the foundations information channels.
Ethical Principles of Partnership

Through the course of the PatientPartner project, a set of Ethical Principles was devised in order to ensure that the integrity of all parties concerned is upheld. The following eight principles were under consultation at time of printing however they describe the base principles necessary for a successful partnership. The full and final report along with a memorandum of understanding will be accessible on the website: www.patientpartner-europe.eu.

Mutual Respect
Central to all partnerships between Patient Organisations and other stakeholders in clinical research is that all parties in the partnership act in accordance with the principles of mutual respect. This means that all partners’ competence, capabilities, and limitations shall be taken into account and respected. Sponsors and investigators should know, understand and respect the environment and constraints within which patient groups work.

Trust
Partnership between Patient Organisations, industry, clinicians and all other stakeholders involved must be based on mutual trust. This trust should stem from openness about motives and the confidence that all parties are working towards a common goal, even if the approaches are made from different perspectives.

Integrity and Credibility
The integrity, credibility and independence of all involved partners, as well as the constraints and obligations under which all stakeholders operate should be respected at all times when negotiating the terms of any partnership.
Reliability
In order to ensure a fruitful partnership, it is important that terms of agreement are set up at the beginning of the collaboration and that these are adhered to throughout the entire research process. We recommend periodic reviews that will evaluate the progress based on the goals and objectives.

It is also advisable to agree up front how conflicts will be resolved and what are the terms for the readjustment or termination of partnership.

Accountability
It is preferable to outline in the early stages of the partnership development how each party will be held accountable for its respective input and the outcomes achieved through the collaboration. The ways in which parties will report back to their members, colleagues, partners and general public should be determined at the start.

Acknowledgement
Agreement as to how each party will be acknowledged for its contribution should be reached before the start of any collaboration. Ownership and intellectual property rights of materials produced in the collaboration should be agreed upon, taking into consideration that Patient Organisations do not necessarily have access to legal advisors and therefore need the terms to be set out in clear and simple language. Endorsement should also be discussed along with the terms of usage of the name, brand and/or logo of all parties including the names of the representatives.
Transparency
Transparency means ensuring that all parties are clear about each partner’s role and responsibility within the partnership. Resources contributed by each party should be used appropriately and any other collaboration that might influence the partnership should be disclosed.

Sustainability
Collaborations should strive for a sustainable benefit for patients, rather than aiming for short-term goals or competitive advantage. This sustainability can be achieved by ensuring that the demands on administrative efforts from Patient Organisations be minimised so that the limited resources can be utilised in other areas. Results of a trial should also aim to be made public after the conclusion of the collaboration to create value for the whole community rather than just competitive advantage for the partners.
Other Clinical Research

Biobanking
What is a Biobank?
In a Biobank, also known as a biorepository, biological materials and the data associated with those materials are collected, stored, processed and distributed. The purpose can be scientific research or medical treatment. Typically these “biological materials” are human samples such as tissue (including organs) or blood and other body fluids, and the “data” is any information including medical information pertaining to the donor of that biospecimen.

Biobanks exist within a variety of institutions including academic medical institutions and pharmaceutical and biotechnology companies. They can also be stand-alone organisations including independent companies (both for and non-profit) that can provide Biobanking services and access to samples as a service to the research community or patients.

For further information on Biobanks EGAN has produced an “FAQ on Biobanks” in English and German which is accessible from their biomeninvo4all website: www.biomedinvo4all.com/en/publications/faq-on-biobanks-various-languages.

How to join a Biobank
When giving informed consent for a clinical trial, a person may also be given the opportunity to agree separately to participate in a Biobank or to decline such participation. Generally, a person will be asked directly to participate in this research by their health care provider.

If someone wishes to proactively volunteer, then the best source of information on Biobanks operating in their local area is the internet. The BBMRI has created a portal which gives access to a list of Biobanks which can be searched using various criteria. To access the portal: www.bbmriportal.eu enter the following: Login: guest, password: catalogue
Registries

What is a registry?
A registry contains personal and medical information on healthy individuals or patients. The information that can be stored includes:
- Personal characteristics of the patient: age, gender etc.
- Disease history of the patient and current diagnosis (including, if relevant, the specific gene mutation that has contributed to the development of the disease)
- Treatments and medication given
- Clinical outcomes of treatment

There are different types of registries depending on their ownership, volunteer group and size.

Registries can be used to gain an overview of the patient population and its characteristics which is useful when companies or academics are planning a clinical trial.

How is it different from a Biobank?
A patient registry is not the same thing as a Biobank, but they can be complementary as they contain information that is mutually helpful in carrying out research. A Biobank will contain human tissue samples and/or a variety of body fluids. A patient registry will have data and information on patients, patient populations, patient groups and sub-groups, as well as general health care information and hereditary data but no body material. Information between a patient registry and a Biobank can usually only be shared following patient consent and when subject to the limitations imposed by appropriate ethical review.

For more information on Registries please see the EGAN/Roche produced “FAQ on Registries” available on the biomedinvo4all website: www.biomendinvo4all.com/en/publications.
Paediatric Research

What is Paediatric Research?
Paediatric research is research involving children. Regulation (EC) 1901/2006 states that all new medicines applying for marketing authorization, which are intended to be used on the paediatric population must provide results from a previously agreed Paediatric Investigation Plan (PIP). The PIP sets out the studies that will be conducted to ensure the product is safe for paediatric use. The regulation also includes incentives for companies to develop medicines for use in children in order to stimulate paediatric developments.

How to get involved in Paediatric Research
Clinical trials involving children are advertised using the same channels as adult clinical trials. What participants must bear in mind is that laws concerning the involvement of children in research differ in every European country. The age of adulthood varies from 16 to 18 meaning that in some countries 17 year old adolescents may require their parents or legal guardian to sign the informed consent form on their behalf.

In the UK, young persons are also involved in specialist advisory groups. There are several groups, each group comprising of approximately 10-15 young members, aged between 8-19 years who have experience of living with a childhood condition, illness, disability or taking medicines.
(For more information visit the MCRN website www.mcrn.org.uk.)

For descriptions of any paediatric clinical trial with investigator sites in the European Union and any trials which form part of a paediatric investigation plan (PIP) including those where the investigator sites are outside the European Union please visit the EU clinical trials register website: www.clinicaltrialsregister.eu.

Registries

What is a registry?
A registry contains personal and medical information on healthy individuals or patients. The information that can be stored includes:
- Personal characteristics of the patient: age, gender etc.
- Disease history of the patient and current diagnosis (including, if relevant, the specific gene mutation that has contributed to the development of the disease)
- Treatments and medication given
- Clinical outcomes of treatment

There are different types of registries depending on their ownership, volunteer group and size.

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Glossary

For a more complete glossary of terms used in research please refer to: www.eurordis.org/IMG/pdf/CT_GLOSSARY_FINAL.pdf

Declaration of Helsinki — ethical standards for research involving human beings, human material or identifiable data, devised by the World Medical Association.

Ethics Committee— Group made up of individuals representing a wide spectrum of society, who are appointed to make decision regarding the ethical aspects of a trial.

Experiential Knowledge — the unique perspective gained by individuals who live with or help care for those affected by a medical condition. Called experiential knowledge because it is knowledge acquired through experience rather than studied.

Pharmacokinetics — The processes (in a living organism) of absorption, distribution, metabolism, and excretion of a drug or vaccine.

Pharmacology — the study of how living tissues and organisms are modified by chemical substances and the interaction between them, i.e. the effects of drugs on the body and the effects of the body on drugs.

Pharmacovigilance — the science of collecting, monitoring, researching, assessing and evaluating information from healthcare providers and patients on the adverse and long term effects of medications.
**Placebo** – an inactive pill, liquid, or powder that has no treatment value.

**Protocol** – The study design which outlines amongst other things, how the trial will take place, what methods will be used, who will be involved and how the results will be reported.
Useful Resources

**BBMRI** (Biobanking and Biomolecular Resources Research Infrastructure) www.bbmri.eu
A pan-European and internationally broadly accessible research infrastructure, and network of existing and new Biobanks and biomolecular resources.

**Biomedinvo4all** www.biomedinvo4all.com
Website created by **EGAN** to provide information on genetic, biomedical, pharmaceutical and clinical research for the development of therapeutic, preventive and diagnostic tools.

**Clinical Trials.gov** clinicaltrials.gov
A service of the U.S national Institutes of Health which allows users to search clinical trials taking place in the US and abroad.

**ECRIN** (European Clinical Research Infrastructures Network) www.ecrin.org
The European Clinical Research Infrastructures Network (ECRIN) is a sustainable, not-for-profit infrastructure supporting multinational clinical research projects in Europe.

**EFGCP** (European Forum for Good Clinical Practice) www.efgcp.be
Is a non-profit organisation established by and for individuals with a professional involvement in the conduct of biomedical research. Its purpose is to promote good clinical practice and encourage the practice of common, high-quality standards in all stages of biomedical research throughout Europe.

**EFPIA** (European Federation of Pharmaceutical companies and Associations) www.efpia.org
Represents 31 national associations and 40 leading pharmaceutical companies operating in Europe.
EGAN (European Genetic Alliances Network) www.egan.eu
Patients Network for Medical Research and Health - is a dynamic collaboration of Patient Organisations that work together because they recognize the value of their involvement in genetics, genomics and medical biotechnology for the prevention and treatment of genetic, multifactorial and congenital disorders.

EMA (European Medicines Agency) www.ema.europa.eu
Agency responsible for the scientific evaluation of medicines developed by pharmaceutical companies for use in the European Union.

EU Clinical Trials Register www.clinicaltrialsregister.eu
Public access database of clinical trials taking place in Europe which also contains a list of sponsor contact information.

Genetic Alliance UK www.geneticalliance.org.uk
A national charity of Patient Organisations with a membership of over 150 charities supporting all those affected by genetic disorders.

INOLVE www.invo.org.uk
Involve are specialists in public participation; bringing institutions, communities and citizens together to discuss, decide and reshape the things that matter to them. Involve makes a practical difference to democracy by delivering, researching and promoting high quality public participation processes.

MCRN (Medicines for Children Research Network) www.mcrn.org.uk
MCRN was created in 2005 to improve the co-ordination, speed and quality of randomised controlled trials and other well designed studies of medicines for children and adolescents, including those for prevention, diagnosis and treatment.
**NCRN** (National Cancer Research Network) [www.ncrn.org.uk](http://www.ncrn.org.uk)
Established by the Department of Health in April 2001 to provide the NHS with an infrastructure to support prospective trials of cancer treatments and other well-designed studies and to integrate and support research undertaken by cancer charities.

**PatientPartner** [www.patientpartner-europe.eu](http://www.patientpartner-europe.eu)
Project set out to promote the role of Patient Organisations in the clinical trials context. See “About this guide” (p.5) for more information.

**People in Research** [www.peopleinresearch.org](http://www.peopleinresearch.org)
Website set up by INVOLVE and the National Health Service to advertise opportunities for public involvement in clinical research.

**Orphanet** [www.orpha.net](http://www.orpha.net)
Orphanet includes a Professional Encyclopaedia, which is expert-authored and peer-reviewed, a Patient Encyclopaedia and a Directory of expert Services. This Directory includes information on relevant centres of expertise, clinical laboratories, research activities and Patient Organisations.

**Roche** – [www.roche.com](http://www.roche.com)
International research-focused healthcare company that produces Pharmaceuticals, solutions for diagnostics and products for researchers.

**Value +**
Project conducted by European Patients’ Forum (EPF) in 2008-2010, to promote patients’ involvement in EU supported health-related projects. The project produced a Handbook, Toolkit and a set of Policy recommendations. These can be accessed from the EPF website: [www.eu-patient.eu/Initatives-Policy/Projects/EPF-led-EU-Projects/ValuePlus](http://www.eu-patient.eu/Initatives-Policy/Projects/EPF-led-EU-Projects/ValuePlus).
PatientPartner

VSOP (Dutch genetic alliance) www.vsop.nl
Is an umbrella organisation of about 55 national, disease-linked, parent and Patient Organisations whose mission is to optimise the implications of research in the field of genetics, medical biotechnology and life sciences for both patients and the public.

World Medical Association www.wma.net
The purpose of the World Medical Association is to achieve the highest international standards in Medical Education, Medical Science, Medical Art and Medical Ethics, and Health Care for all people in the world.
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