CLINICAL TRIAL AND RESEARCH

Information for patients

SERVIZIO SANITARIO REGIONALE
EMILIA-ROMAGNA
Azienda Unità Sanitaria Locale di Reggio Emilia
IRCCS Istituto in tecnologie avanzate e modelli assistenziali in oncologia

Summary

Introduction

The Scientific Institute for Research, Hospitalisation and Healthcare (*IRCCS*) in Advanced Technologies and Care Models in Oncology in Reggio Emilia is a centre of excellence in the field of oncology. Recognised as an IRCCS by the Ministry of Health since 2011, it is part of the Local Health Authority (*LHA*) of Reggio Emilia and is based at the Arcispedale Santa Maria Nuova Hospital and the CORE - Oncology and Haematology Centre of Reggio Emilia. The IRCCS of Reggio Emilia provides assistance by ensuring outstanding cancer prevention, diagnosis, care and rehabilitation. It also conducts clinical, translational and healthcare cancer research.

The clinical research includes projects conducted on patients being cared for by the LHA-IRCCS. The general goal is to study patients in all aspects, focusing on diagnosis, treatment and follow-ups. Professionals from various disciplines work as a team to provide an overview of the patient that takes into account the complexity of the cancer and helps develop and assess personalised medicine courses.
The aim of translational research is to transfer the results obtained in the laboratory to the patient's bed. Translational research projects seek to improve our understanding of the molecular mechanisms behind cancer so we can develop new instruments for the diagnosis, prognosis and care of patients.
Healthcare research assesses the quality of cancer support and the clinical, organisational and economic impact of complex operations aimed at improving the quality of services. It also assesses the impact of innovative technologies on clinical outcomes and organisational contexts.
Aim of this leaflet is to explain the main aspects of clinical trials and patients' participation in the trials.

CLINICAL TRIALS: WHAT YOU NEED TO KNOW

What's a clinical trial?

It is a research study which identifies theeffectiveness and safety of new drugs, treatments or medical interventions. Clinical trials can help to decide if a new treatment turns out to be more cost-convenient, whencompared to an already available one. "Clinical" means that tests are performed on humans, patients or healthy volunteers. Clinical trials are usually anticipated by a preclinical phase, when in vitro or in vivo tests are performed, respectively on cell-cultures (*in vitro models*) or test-animals (*in vivo models*).

What does experimental treatment mean?

It means: new treatment whose effectiveness and risks don't possess enough scientific evidence. Aim of a clinical trial is exactly to assess the effectiveness and safety of an experimental therapy.

What's a clinical trial protocol?

It is a document which defines the trial in detail; it gives a full description of:

- objectives;
- investigators' scientific rationale;
- participants' predetermined characteristics which are necessary to partecipate in the trial (*so called inclusion or exclusion criteria*);
- number of participants allowed to take part;
- exams to be performed by participants and their due date;
- way of performing experimental treatment;
- way of assessing the treatment effectiveness;
- statistics used in analysing the results of clinical trials.

How many types of clinical trials are there?

Clinical trials have different purposes: diagnosis, prevention, treatment of diseases. The following list describes the main types of clinical studies and their objectives:

- **screening trials** evaluate the test capability of detecting a disease before it provokes symptoms;
- **diagnostic trials** assess the capability of tests or procedures to diagnose a particular disease (*sensitivity*) and to detect a disease only when it is really present (*specificity*);
- **treatment trials** assess the effectiveness of new drugs or more generally of new treatments;
- **quality of life trials** assess how diseases or medical interventions impact on different aspects (*including the psychological one*) of ill persons' daily life.

Moreover, clinical trials vary in relation to their methodology. The following description briefly explains some of the main types of clinical trials.

Controlled clinical trial

Participants are divided into two groups: in one group, participants receive medical intervention or experimental treatment, in the other one (*so calledcontrol group or simply control*) they receive no treatment or placebo*. This kind of procedure allows to compare the course of disease in both groups and to assess the effectiveness of treatment under examination.

*Placebo: is a simulated or otherwise medically ineffectual disease treatment. Placebo is given as pill, tablet or liquid to patients belonging to the so-called "control group". Placebo is used to verify whether the effects detected in patients receiving treatment (*for instance a medication*) are due to their expectancy of healing or improvement and not to the effectiveness of clinical trial.

Randomized controlled trial

It is a type of trial, whose participants are randomly allocated: either to the group receiving medical treatment or to the control group. Usually participants are allocated through a computer programme, in order to ensure a comparability between the two groups.

Blind and double-blind studies

In blind trials patients, unlike investigators, don't know which group they belong to. In this case, patient doesnt'n know whether he will be treated with experimental treatment or with placebo. In double-blind trials, neither patients nor investigators know who is treated with experimental treatment and who with placebo.

Multicenter research trial

In multicenter research trials, many hospitals or research centers take part to the trial. In case hospitals belong to the same country, trial is described as national trial while, if hospitals belong to different countries, trial is described as national trial.

What are the phases of clinical trials?

A clinical study may last several years; during its course, experimental treatment undergoes a series of assessments according to well defined rules. For this reason, clinical trials go through different phases. For instance, in case of testing new drugs:

- **Phase I clinical trials**: their aim is to fix the maximum tolerated dose of a drug and the most appropriate dose, in comparison to the side effects it causes. Some Phase I clinical trials also assess tolerability in case of a new route of administration related to a given drug (*for instance, injection instead of by mouth*).
- **Phase II clinical trials** assess the effectiveness of a treatment and record possibile side-effects. To evaluate effectiveness, the following points are taken into account:
  1. how many patients benefited from the treatment (*totally or partially*);
  2. how long do these benefits last (*time of absence of disease*);
  3. patients' median survival.
- **Phase III clinical trials** compare a new treatment with a standard one, taking into account both effectiveness and side effects.
- **Phase IV or "post marketing surveillance"** clinical trials: drug is already on the market, but further information is collected, concerning the drug's effectiveness, risks arising from its use and what's its best use.

Are there only clinical trials?

No. There are other types of trials leading neither to direct intervention in patients nor to their participation in those trials. They may be observational and epidemiological trials, or data collections: labs, genetics, genomic, pharmacogenetics.

What's an Ethics Committee?

Those who choose to participate in a clinical trial are adequately protected. All study protocols conducted at the LHA-IRCCS are approved in advance and monitored by the competent Ethics Committee and the regulatory authority in the cases provided for by law, in order to ensure that risks are minimised and are less than the potential benefits.

The Ethics Committee is the body responsible for safeguarding patients' rights and is an independent body, whose minimum composition is established by law. The composition of Ethics Committees must ensure the qualifications and experience necessary to evaluate the ethical, scientific and methodological aspects of the proposed studies and to perform the functions assigned to them. The members of Ethics Committees must have documented knowledge and experience in clinical trials of medicinal products and medical devices and in other matters within the competence of the Ethics Committee.

The Competent Regulatory Authority is the institution that, at national level, protects patients participating in a clinical trial, ensuring that it is conducted safely and with all the guarantees provided for by European and Italian legislation. It varies depending on the type of trial. In Italy, the main competent authorities are the Italian Medicines Agency (*AIFA*), the Ministry of Health and the National Institute of Health (*ISS*).

No study may be initiated at the LHA-IRCCS unless it has received a favourable opinion from the competent Ethics Committee and the Regulatory Authority, where applicable, following an assessment of the scientific merit of the study protocol, its scientific relevance, the risk/benefit ratio, statistical analysis, feasibility and ethics of the study, and unless it has received clearance from the LHA-IRCCS.

Who sponsors clinical trials?

Clinical trials are sponsored by foundations, research institutes in public or private services, hospitals, pharmaceutical companies, patients' organizations.

TAKING PART TO CLINICAL TRIALS

Who can partecipate in clinical trials?

In order to have useful results, clinical trials are conducted on groups of people with similar physical characteristics.
For this reason, each clinical trial protocol specifies:

- **inclusion criteria** are features that the prospective subjects must have if they are to be included in the clinical trial;
- **exclusion criteria** are features that, if possessed by prospective subjects, prevent them from being included in the clinical trial.

The following characteristics are usually taken into account:

- age (*young, adult, old people*);
- sex *(women are kept out of some trials to avoid harmful effects on foetus*);
- diagnosis;
- phases of disease;
- comorbidity.

Benefits of participating in clinical trials

- To learn personally how to take care of your health;
- To have some treatments available before they become such for patients in general;
- To be treated by expert professionals in research and healthcare, and to take an active role in the development of scientific research.

Risks and/or disadvantages of taking part to clinical trials

In some cases, the safety and effectiveness of experimental treatments may be unknown. Usually risks are not higher than those connected to other medical treatments and to the development of a disease. Sometimes, the suggested treatment may induce troublesome side effects, even severe ones. Participation in clinical trials often requires a longer time, in comparison to conventional treatments, as it is necessary to undergo more frequent exams and to spend longertime in hospital.

Is it possible for a participant to withdraw from the trial?

Yes, patients may withdraw from a trial at any time and for whatever reason.

What is "informed consent"?

In order to perform whatever healthcare intervention, with exception of those connected to emergency situations, patients must give their permission. They may freely accept to partecipate in a clinical trial (*clinical or observational trial*) by signing a document, the so called "informed consent", which must be validated by the Ethics Committee,before it is used. Informed consent is the same for all participants to the same clinical trial. It consists of:

- a full description of all information related to the proposed trial;
- a form to be signed by the patient accepting to take part to the trial.

Before getting patient's signature, investigator must give him all information connected to the trial. Moreover, investigators must ensure that patients have adequate comprehension of the meaning oftheir decision. Informed consent must be personally signed and dated, both by patient and investigator who informed the patient.

Some questions a patient must raise before taking part to a clinical trial

First of all, before deciding to partecipate in a trial, patient must carefully read all pertinent written documentation he has been given and ask the investigator to clarify any points that may seem unclear. Questions posed by patients can ease their communication with investigators:

- What is the purpose of the trial?
- Why do investigators believe the experimental treatment being tested may be effective?
- Has the treatment been tested before?
- If so, what's the result given by other studies?
- What are the side effects connected to the treatment?
- How long will the trial last?
- Is there any effective alternative treatment available nowadays? Is it equally effective?
- Will hospitalization be required?
- Who will sponsor the experimental treatment?
- Will my personal and sensitive data be preserved?
- In case the proposed treatment turns out to be effective, will I receive it free of charge when the study ends?
- Will I be reimbursed for the costs I have incurred?

Edited by the Scientific Directorate

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