IFPA
GLOBAL LEADER IN FIGHTING PSORIATIC DISEASE

GLOSSARY

Common terms in clinical research

Understanding the language of clinical trials

BREAKING BARRIERS

Why does this matter?

Recognizing and understanding the language used in clinical research is essential to increasing participation and adherence to studies.

In this glossary, we have compiled information on clinical research and a list of terms that are often used but may not be fully understood by the general population and, most importantly, by the people who may participate in the study.

This document will help you understand trial characteristics, how they are planned, the language used and what may be asked of you as a potential trial participant.

The clinical study life cycle usually follows the following steps:

1 Study design and registration

2 Participant enrollment

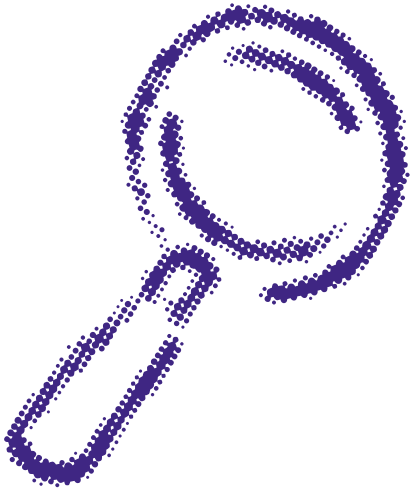
3 Study completion

4 Publication

5 Regulatory application

In this document, we will follow this structure to introduce the different concepts and terms.

Observational



This type of study does not involve any active intervention, such as treatment or changes in lifestyle. Instead, participants in the study are followed over a period of time to see how their health naturally changes (or not) over time.

Registries

Registries are prospective long-term observational cohorts created to help researchers learn how, for example, treatment performed in real-world care over time.

Example of registries:

- **BADBIR** – UK and Ireland registry
- **PsoBest** – German registry
- **PSOLAR** – Multi-national registry
- **PURE Registry** – Canada and Latin America registry
- **KPR** – Kenyan Psoriasis Registry
- **WJPR** – Western Japan Psoriasis Registry
- **ADR** – Australian Dermatology Registry (for several dermatological diseases)

Cohort studies

The simpler type of observational study. Cohorts, or groups of people, are followed over time to observe a specific outcome. In this type of study, the cohort can be divided into groups based on certain characteristics and the groups are compared over time.

Cohort studies can be retrospective or prospective.

Example of a prospective study: *recruit a group of healthy people according to specific requirements, document medical events and observe how many develop psoriasis over time.*

Example of a retrospective study: *identify people with psoriatic arthritis that experienced a heart attack, in comparison to people with psoriatic arthritis without any cardiac event history and look back at all medical information to find possible factors that may correlate with the myocardial infarction.*

Case-control studies

Two cohorts are created – one is the case and one is the control – and both are investigated. This type of study is performed at a specific moment in time and designed to identify health information that is common within a group of participants and different from a control group.

Example: *evaluate the impact of geographic setting on psoriasis severity by comparing two populations, one living at the beach and one in the mountain. This study would help to understand if environmental factors are associated with the severity of psoriasis.*

Cross-sectional studies

Performed at a specific moment in time and designed to identify health information common within a group of participants and different from a control group. Participants are not followed over time.

Example: *study the prevalence of comorbidities in a group of people living with psoriatic disease compared to a group of people without the disease. This type of study evaluates if psoriasis affects the susceptibility of developing comorbidities.*

Longitudinal studies

This type of study follows participants over long periods of time – years or even decades – with repeated monitoring during the study.

Type of clinical research studies

Interventional

This type of study is associated with a particular intervention or treatment.

What are interventions?

An intervention is any treatment, action, or approach being tested to find if it is safe and improves health.

Pilot studies

A smaller study with a limited number of participants, sometimes conducted before a large trial. These studies can answer different questions, such as whether it is possible to conduct the study on a large scale, whether the protocol is correct or needs to be changed, and whether the outcomes being measured are adequate, among others.

Treatment studies

Commonly called clinical trials. Designed to compare:

Intervention versus Control: A new treatment(s) compared to no treatment (or placebo).

Intervention versus Intervention: When all arms of the study receive some form of treatment (the control is not a placebo).

Examples include: *A new treatment or combination of treatments compared with an already established treatment; a different treatment dosage or frequency of administration compared with the standard protocol; a lifestyle habit compared with a different lifestyle habit, such as a low-calorie diet compared with a high-calorie diet.*

Clinical research usually falls into two types of studies that can overlap:

Quantitative study: A study in which outcomes or parameters being measured are numerical and well defined, for example yes/no, PASI (Psoriasis Area and Severity Index), blood pressure, glucose levels.

Qualitative study: A study that aims to understand meanings, experiences, and perspectives through descriptions. This type of study is aimed at understanding patient experiences. These can be assessed through patient surveys or personal interviews, for example.

Clinical trial groups

The different groups in the trials can also be called ***arms***, and there can be multiple arms in one study.

Every study contains at least one interventional and one control or placebo group, and both are compared.

The ***intervention*** is the specific modification or treatment being administered to the participant, and which effectiveness and safety are being tested.

The ***control group*** can be, for example, a placebo (no treatment) or a previously approved treatment.

Placebo means that no active treatment is given. If the study is testing a new pill, the placebo can be a sugar pill that is similar in appearance and form to the treatment pill; if the treatment is a new injectable, the placebo can be a saline injection (which has no active ingredients). The placebo helps establish the natural progression of the disease being studied unrelated to any intervention.

Clinical trial phases

Clinical trials go through 4 phases, and only if the previous phase shows positive results can the study move to the next phase.

Phase I

A small number of healthy participants are recruited to test the safety of a new drug. Safety means that it is not harmful.

Phase II

A larger number of people living with the disease are invited to participate in the study to confirm the optimal dose and detect any adverse events related to the drug being tested.

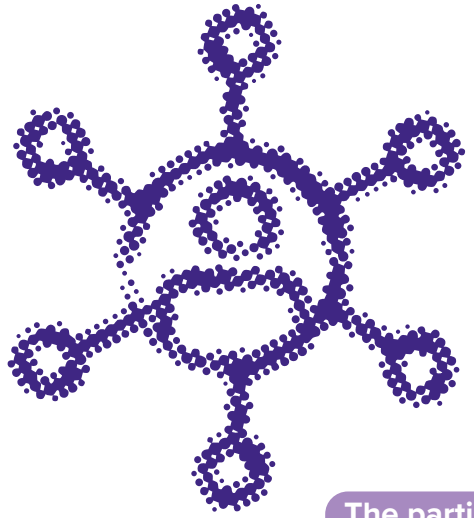
Phase III

Between hundreds and thousands of participants are recruited for each arm of the study. This phase is designed to confirm the efficacy and effectiveness of the drug compared with current treatment options or no treatment, depending on the study. If the results are positive, an application for approval is submitted to the regulatory agencies.

Phase IV

This phase is conducted after the drug is approved. Continuous participation of patients and monitoring of long-term safety and efficacy of the drug in routine clinical practice. This is also called real-world evidence.

Key stakeholders in clinical trials



Who is part of the study site team

- **The principal investigator**
  The responsible leader of the team
- **Sub-investigators**
  For example medical doctors, who perform trial activities
- **The clinical research coordinator**
  Oversees the logistics of the trial and ensures documentation requirements are being followed
- **The research or study nurse**
  Conducts most participant-facing tasks.

The participant

The person taking part in the study. Sometimes the participant can be called study subject.

The trial site

The location or locations where the trial takes place, often a hospital or a clinic.

The study site team

The people involved in planning, conducting, and final reporting of the study results. They are qualified professionals with experience in conducting clinical studies. The team is multidisciplinary and may vary depending on the studies.

The funder or sponsor

The group, agency or company that initiates, monitors, and funds the clinical trial. They have the responsibility to ensure the safety of the study, including the treatments, and to report the results.

Ethics committee or Institutional Review Board

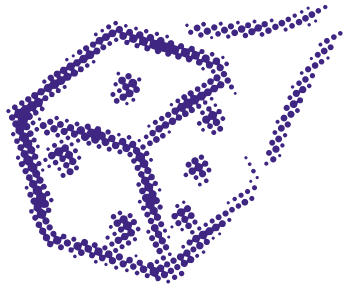
Group of people with relevant competence who ensures the proposed study can be safely performed. The experts are independent and are not involved in the study itself. The study protocol is evaluated to ensure that it follows good clinical practices, protects participants' rights and safety, and that the results are scientifically and/or medically valid.

Regulatory agency

An official governmental body that regulates and approves, for example, new drugs to treat specific symptoms or diseases. Approval is granted when studies conducted on the drug demonstrate clear health benefits and safety for those who will take it. Approval is given after Phase III trials are successfully completed. The agency continues to monitor safety after approval and has the authority to recall or stop the prescription of the drug if new risks are discovered.

Examples of Regulatory Agencies:
*- **FDA**: Food and Drug Administration, in the USA*
*- **EMA**: European Medicines Agency, in the European Union*

Clinical trial characteristics

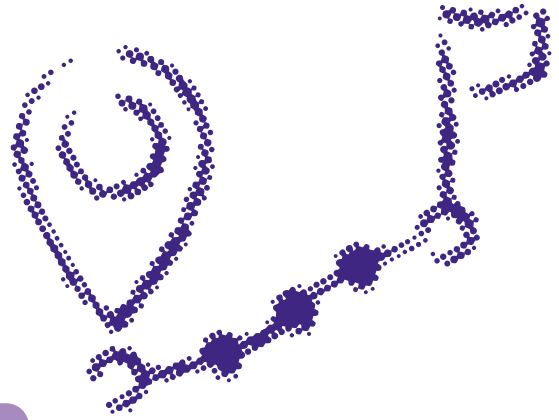


Randomization

Randomization ensures that people in a clinical trial are put into different treatment groups by chance, similar to flipping a coin. This helps make sure the results are unbiased.
To keep the groups balanced, researchers make sure that important factors – such as age, gender, severity of disease or past treatments – are evenly represented in each group.
Randomization is an important requisite in phase 3 trials and helps researchers understand whether a treatment truly works and is safe.

Blindness (sometimes called masking)

Blinding a trial means that the person or persons involved do not know which arm they are part of. Studies are single blind when only the participant does not know which arm they are part of. Studies can also be double blind, when both the participant and the clinical trial team (researcher, health care professional) do not know which arms of the study the participants belong to. The information on the arms is only disclosed at the end of the study, when the results are analyzed.



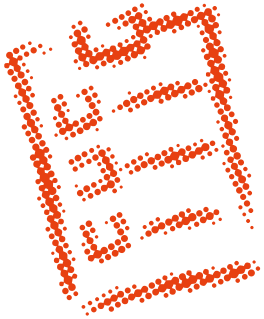
Baseline

The starting point of the study. Often refers to the medical measures related to the study participant at the start of the study. This information is compared with the information at different time points during the study, for example 12 weeks after enrollment in the study. Some baseline characteristics include demographic attributes (age, gender, body weight), BSA (Body Surface Area) and PASI (Psoriasis Area and Severity Index), comorbidities, disease duration, treatment history and others. The characteristics and patient reported outcome questionnaires (PROMs) used at the endpoints will also be assessed before the study starts.

Clinical trial characteristics

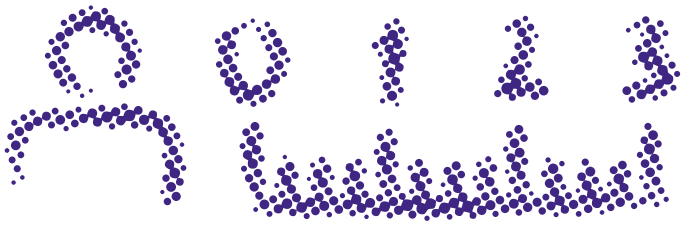
Study protocol

The protocol is a detailed explanation of how the study will be conducted.



Sample size

During the planning of the study, the research team determines how many participants need to be recruited. An adequate sample size ensures that the study is both clinically and statistically valid, while also preventing over-recruitment. Avoiding unnecessary enrollment is important, as exposing more individuals than needed to the study drug would be unethical.



Clinical trial registry

A publicly accessible database that lists planned, ongoing, and completed clinical studies. It provides key information about each study, including the study objectives, sponsor or principal investigator, eligibility criteria, study design and outcomes, and supporting documentation. Clinical trials are required to be registered in a registry before recruitment begins. Example of such registries include clinicaltrials.gov and euclinicaltrials.eu.

Participants

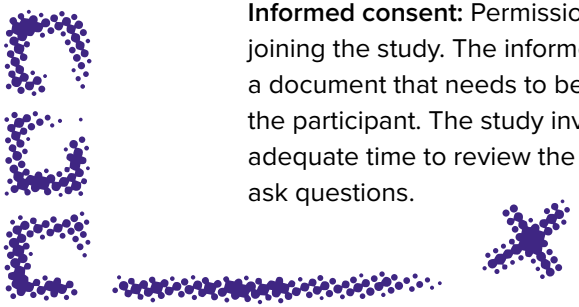
Recruitment

Process by which the study participants are enrolled or accepted in the study.

It includes identifying individuals that may participate in the study, informing them about the study goals, evaluating if the eligibility criteria are met, presenting and obtaining the informed consent. The individual is considered as part of the study once they have signed the consent form.

Consent

Informed consent: Permission given by the participant before joining the study. The informed consent form is presented as a document that needs to be understood and signed by the participant. The study investigator should give the patient adequate time to review the informed consent form and ask questions.



What is included in an informed consent form?

The consent form includes all the important information regarding participation in the study:

- The purpose of the study
- The study characteristics, for example what is the new treatment being studied and how is it going to be tested
- What is required from the participants in the study, for example: time commitment, number of visits to the trial center, types of tests and samples required and when they will be collected, whether any medication or lifestyle habits need to be changed, etc.
- The study duration
- The risks of participating in the study, including any side effects – ranging from common to rare – and their likelihood of occurrence, as well as the risks of any procedures to be performed, such as biopsies or other invasive tests.
- What are the benefits of participating in the study
- Financial compensation, if applicable
- Confidential information, for example, patient information (name, address, contact information, and any identifiable information) is collected, but a patient code is created and used in the study instead. Before the publication of the study, any personal information belonging to the participants is removed or transformed to protect their privacy.
- The rights of the participants, for example, the right to withdraw from the study at any time
- What personal information will be collected and how it will be used and saved
- Study recruiters' contact information

Participants

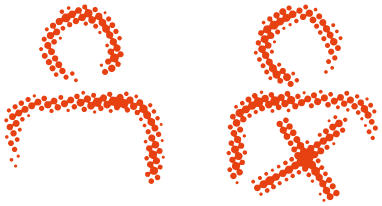
Screening

In order for individuals to participate in a study, a detailed evaluation is conducted.

Eligibility criteria: Rules that determine who can join the study. These are usually a list of inclusion and exclusion criteria, or participant characteristics that determine if the candidate can or cannot join the study. Examples of criteria are age, gender, pregnancy, disease characteristics, treatment history, comorbidities, ethnicity, lifestyle habits, etc.

Inclusion criteria: Information that makes an individual a good candidate to participate in the study.

Exclusion criteria: Information about an individual that disqualifies him/her from participating in the study.



Retention or withdrawal

Retention: The ability to keep the study participants enrolled in the study until it is completed.

Withdrawal: When a participant leaves the study before it is completed. Importantly, participants can withdraw from the study at any given point, even immediately after signing the consent form. Withdrawal can also occur if the enrollment needs are not met or if the participant retention is low. Withdrawal by the study organizer can occur before the study starts.

Follow up

According to the study plan and the informed consent form, participants are required to visit the clinical trial site to monitor their health over time. This can be done through a combination of assessments with a healthcare professional, filling out questionnaires or surveys, and other methods.

End of study

Clinical trials have an estimated completion date. This date represents the time at which the last participant has been assessed according to the outcome measures, primary and secondary endpoints and adverse events set up in the study plan.

Outcomes

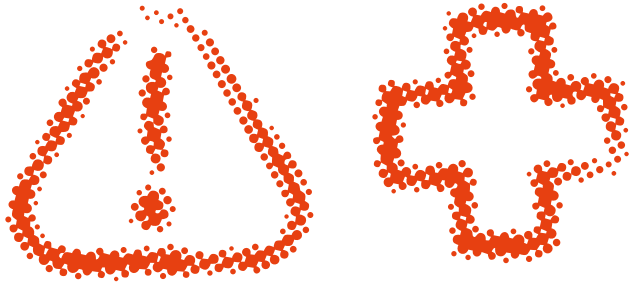
Outcomes are the measurable results used to evaluate whether an intervention is effective and safe. For example, a new treatment for psoriasis may be considered successful if it clears skin flares and reduces itching.

Patient reported outcome measures (PROMs)

These are standardized forms or questionnaires that the patient fills out about their health. PROMs capture the patient's experience, including symptoms, feelings, health-related quality of life, and thoughts on treatment. They can be disease-specific, such as the Psoriasis Disability Index or the Psoriasis Quality of Life Questionnaire, or more general, designed to assess overall health and well-being and applicable across different diseases, such as the Short Form Health Survey 36 or other Health-Related Quality of Life questionnaires.

Adverse or serious adverse event

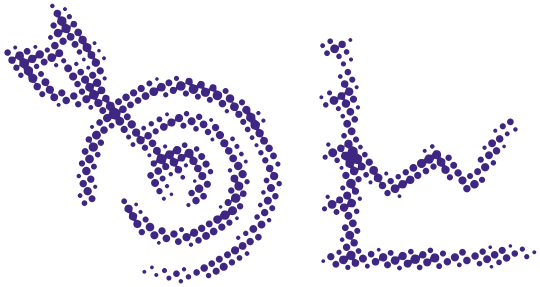
An unwanted medical event that occurs during the study. It may or may not be caused by the treatment being tested. Serious adverse events may require hospitalization or even result in death. These events are reported in the study, to the study sponsor, to the ethics committee, and in any publications arising from the study. If an event is caused by the treatment under investigation, the study protocol may need to be revised, or the study may even be stopped.



Outcomes

Endpoint

An outcome or result that a study aims to achieve. Studies can have one main or *primary endpoint* and one or more *secondary endpoints*. Secondary endpoints are additional outcomes beyond the primary endpoint. In psoriasis, a common primary endpoint is PASI 75/90, which means that at least 75 or 90% of the skin clears with the treatment compared to baseline at the start of the study. Secondary endpoints can include improvement in quality of life, reduction of itch or pain, and disease clearance in specific areas, such as the scalp.

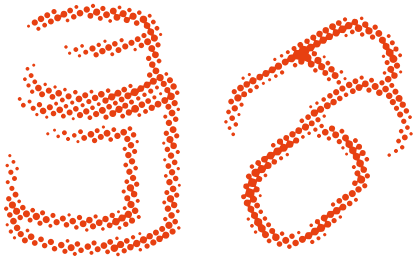


Efficacy

If an intervention or treatment produces the expected result under ideal circumstances, the treatment is said to be efficacious.

Effectiveness

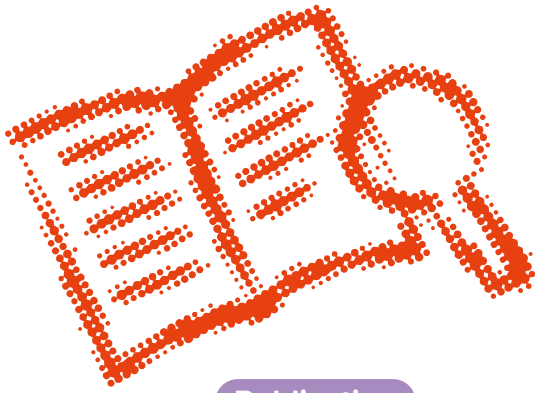
It measures how well an intervention or treatment produces the desired effect, often in routine clinical practice. If the treatment achieves this, it is said to be effective.



Statistical significance

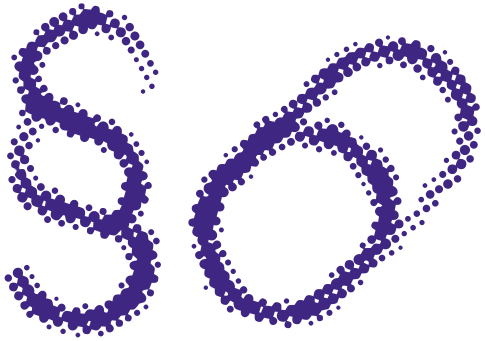
A mathematical way of showing that the differences observed between the study arms (intervention group versus control group) are unlikely to have occurred by chance. In a study testing a new treatment for psoriasis and evaluating skin clearance, statistically significant difference between the treatment and control groups means that the treatment is, with a very high probability, the cause for the differences observed between the groups.

After study completion



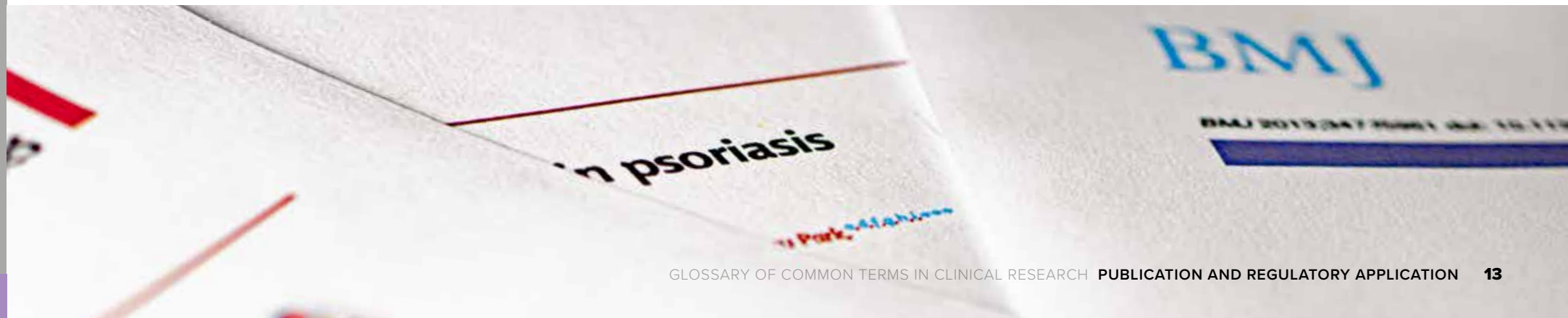
Publication

Clinical trials results are shared with the community through publications. Studies such as clinical trials are published in the form of scientific publications. The process of scientific publication involves other researchers evaluating the study and its conclusions to guarantee the quality and validity of the research. Press releases are often used as tools to share the findings to a wider audience.



Regulatory application

For new drugs, governmental regulatory agencies such as the FDA (USA) or EMA (Europe) are required to review the results of clinical studies. These agencies evaluate whether the drug provides a meaningful benefit to patients and the healthcare system and then decide whether it should be approved for use.



IFPA in brief

IFPA strongly believes that the best way to find information and further resources is to get connected to a patient association. Our priority is to connect IFPA's global members all around the world. Visit IFPA's members page for a list of member associations: ifpa-pso.com.

We encourage you to contact local associations for support in living with psoriatic disease – IFPA continues to stress the importance of the patient-provider relationship when making any treatment decisions and that the patient should remain at the center of decisionmaking processes. The decision to switch between treatments should be made on an individual basis and only with the full, informed consent of both patient and provider.

IFPA is a non-profit organization uniting national and regional associations from around the globe.

At IFPA, we envision a world without suffering from psoriatic disease. To achieve this, we focus on empowering our members, improving living conditions for people living with psoriatic disease and raising awareness.

Visit: ifpa-pso.com

Breaking Barriers

Addressing gaps and reducing stigma and under-representation in clinical research for psoriatic disease

Diversity matters in psoriatic disease research, yet many communities remain underrepresented in clinical trials. Breaking Barriers is IFPA's new global project that explores why participation gaps persist, and how we can close them. Through collaboration among people living with psoriatic disease, researchers, and healthcare professionals, we aim to identify the social, cultural, and structural barriers that prevent inclusion in clinical research and to help remove them. As a part of this project, we have developed accessible educational materials about clinical research and inclusion. Scan the QR code for more information.

IFPA

GLOBAL LEADER IN FIGHTING PSORIATIC DISEASE

www.ifpa-pso.com | info@ifpa-pso.com

BREAKING BARRIERS