

## 2 Introduction

This section may include information about the disease itself, provide some history of other information that already exists, and explain why the current study is different and worthwhile. What problem is this study trying to solve? Why has this research been done, and where does it fit into what is already known? The Introduction is the setup for the rest of the article. It is also a good place to get an overview of a particular problem or challenge about the disease being researched.

### INTRODUCTION

- Background on current study
- Overview of existing studies and findings
- Explanation of how this study is different
- Statement of research questions

## 3 Methods

The Methods section follows the Introduction and explains in detail how the research or clinical study was carried out. In general, studies are either preclinical (trying the drug on animals before testing it on people) or clinical (testing the drug first in healthy people and later in people who have the disease). It's important to know that not all studies are equal or comparable, often because of the methods used. Randomized controlled trials (RCTs) are widely considered to be the most reliable type of clinical study.

**RCT** A study in which participants are placed into treatment groups by chance (randomization), and results are considered against a comparison (or control) group.

## 5 Discussion (cont.)

### DISCUSSION

- Key findings
- Authors' thoughts about the results
- Conclusions that may be drawn from the research
- Limitations of the research
- Ideas or need for future research

Reading clinical publications can be challenging at first, but it becomes easier with practice. Consider reading them with a friend so you can share thoughts and ask questions. Knowledge can be power, and putting in the time and effort to understand clinical publications is one great way to play an active role in your own care and the care of your loved ones. The discussion section is often one of the more simple sections to comprehend as there is no heavy statistical data. This section may be biased as it is based on the authors own conclusions. Please remember to discuss the data and any questions or concerns you may have with your HCP.

### Common Types of Studies

<b>Treatment trials or drug studies</b>	Test new treatments, new combinations of drugs, or new ways of treating a disease or health condition
<b>Diagnostic studies</b>	Decide the best tests or ways to measure or diagnose a disease or other health condition
<b>Quality-of-life studies</b>	See how patients' quality of daily life is affected by their condition, and possibly by disease management interventions
<b>Prevention trials or studies</b>	Look for ways to prevent people from getting a disease, or to stop a disease from coming back
<b>Natural history studies</b>	Watch participants over time to see how a disease affects different people

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# A Guide to Reading a Clinical or Research Publication

Being able to read and understand the information found in a clinical or research publication can be valuable for people who has an interest in his or her own healthcare. It can help people be more knowledgeable about a health condition or disease, be empowered to have active discussions with their healthcare professionals (HCPs), and be more engaged in their own care, regardless of the disease they live with.

But many people find reading clinical publications similar to reading a different language—it just doesn't make sense to them. The good news is that like a new language, the language of clinical publications can be learned, too. The information in this guide will help you understand what the different parts of a clinical publication are, the kind of information they provide, how to make sense of them, and how to use them to increase your knowledge about rare disease and its management. Be sure to discuss any questions or concerns with your HCP.



Developed by Amicus Therapeutics as part of our ongoing commitment to the patient community to enable education in healthcare and research.



## Five Key Sections

There are different kinds of scientific publications, but many are based on results from basic science studies or clinical research trials, exploring whether a medical strategy, treatment, or device is safe and effective. These articles, or clinical papers, are usually arranged into the same 5 key sections: **Abstract**, **Introduction**, **Methods**, **Results**, and **Discussion**.

### 1 Abstract

The Abstract is usually the first section of a clinical publication and often can be a very long (and possibly overwhelming) paragraph. But the Abstract is extremely useful, as it provides a summary of the main objectives (or goals) of the study, how the study was conducted, as well as a concise overview of the results. Keep in mind, however, that the Abstract should never be used as the only source of information about a study because authors have the freedom to choose whatever they think is most important to include in this section. In other words, in some cases, the Abstract might leave out information that you would find important or more relevant. To be fully aware of all the information contained in the article, it is always best to read the entire publication.

#### ABSTRACT

##### One-paragraph summary, including:

- Purpose of the study
- Basic procedures (for example, how the study was conducted, which patients were included, and what procedures or treatments were used)
- Main findings (or results)

### 3 Methods (cont.)

It is worth noting that there are important differences between a rare disease study and a study of a more common disease. Most rare disease studies include fewer participants because fewer people have the disease. In addition, sometimes there is no comparison group because no treatment exists as a “standard of care” to test the new drug or procedure against, and there might be no placebo (inactive control) group because it might be unethical not to provide some treatment to people in the study.

#### METHODS

- How the study was designed
- How data (or study learnings) were collected, organized, and analyzed
- Who was able to participate
- What measures and equipment were used, and when
- What the participants were asked to do and how they did it
- What statistical analyses were used

#### Types of Study Designs

<b>Interventional</b>	Used to understand how a treatment, medicine, or preventive measure affects a disease
<b>Observational</b>	Watches (“observes”) participants to see how their health changes or their disease progresses over time
<b>Meta-analysis</b>	Combines and explores the results of many trials of the same product, or experiments on the same type of participants

### 4 Results

The Results section reports the findings from the study. This section can be highly technical and very informative. The text will explain what happened in the study, while the figures and tables offer additional information. Sometimes figures and tables can be easier to follow.

The Results section will also include a statistical analysis of the study. Key results will often be followed by a “*P* value.” The *P* value represents the likelihood that random chance could explain the result. The publication will tell you what *P* value the authors consider to be “significant.” In many cases this value is less than 0.05 (often shown as  $P < 0.05$ ). If a *P* value is significant, it means that the result was probably caused by more than just chance, so the finding could be more meaningful. If the *P* value is “nonsignificant” (if it has a value higher than what the researchers determined to be significant), it means that random chance could explain the result.

#### RESULTS

- Who completed the study, who did not, and why
- Main findings, including tables and figures
- Statistical importance
  - Significant = Results appear to be caused by more than just chance
  - Nonsignificant = Results could have been caused by chance
  - *P* value = The probability (*P*), or likelihood, that random chance could explain the result. For example the *P* value may be used to highlight whether there was a significant difference between two treatment groups in a study or whether starting treatment resulted in a significant change from prior to initiation of the study

### 5 Discussion

The Discussion is the last section and gives the authors a chance to explain what they think about the findings. The Discussion often includes a list of what the authors think the most important findings are, as well as a list of limitations. It’s important to note that, like the Abstract section, the Discussion section contains the authors’ opinions.