

Introduction to the PFIC.org Research Library

This library contains a number of scientific articles that have been selected based on community interest and suggestions by the scientific board members. We plan to expand this library to provide a good overview of current state-of-the-art research and important historical studies that are intended to assist the community in learning more about PFIC.

Below are a couple of suggestions and explanations that would be good to consider before delving into this educational resource:

- Together with each scientific article we include a brief summary of the article in lay language. The scientific language in articles can be hard to understand, and these summaries are meant to highlight the main points of an article. However, by definition the details of a research study that can be found in the article are often lost in this translation.
- It is important to be aware of the date of publication of an article: findings and conclusions presented in older papers might have been more recently revised or adapted based on newer evidence. This is how science works: it is a continuous effort to increase our knowledge, and this often involves adapting, revising, or even refuting previous findings.
- It is also important to realize that an article is usually written by a limited number of authors. Although published papers in this library are peer-reviewed (meaning they have been checked by colleagues), an article still mainly represents the views of the authors. These views may or may not represent the scientific consensus at the time of publication. Again, this is how science works: publications are an essential part of the scientific discussion. Through this discussion ideas are sharpened, and scientists are forced to come up with good arguments to defend their conclusions.
- A number of the articles in this library are case studies/series. This means that the case of a single patient or small group of patients is described. These studies are very important because they often describe new treatments, or some newly discovered aspect of PFIC. However, since findings of a case study/series are based on a limited number of patients, it may not really possible to generalize the findings: what works for one patient might not work for another.
- Some articles describe large groups of children with a specific disorder. These can be findings amongst multiple centers and consortia of centers. Data can be collected retrospectively (looking backward) or prospectively. Prospective data is often of higher quality as it avoids bias in looking backward at information. Because of the larger number of patients from which the data is developed the findings may be more

accurate and generalizable than case studies or limited case series.

- Some articles in this library are review articles. This means that they provide a summary of the past most relevant research and may include the specific opinions or interpretations of a wide body of research by the author(s). Review articles are often a good way to start learning about a topic.
- An article can also describe a clinical trial. In a typical simple setup for a clinical trial two groups of study participants are matched so that they are as similar as possible in all characteristics. The one group gets treatment 1, and the other treatment 2 (which is often a placebo). Since the groups only differ by which treatment they got, it is possible to decide whether treatment 1 is better than placebo. It's important to understand that if the groups would differ with respect to other characteristics (for instance different mutations of PFIC genes) then different outcomes of the two groups could also be due to the different mutations. That's why sometimes only children with very specific mutations are allowed to participate in a clinical trial.