7th Workshop
Eurordis Round Table of Companies

"Proof of Concept and Level of Evidence in Orphan Drug Development"

December 14th, 2007
Paris, France

La Maison des Arts & Métiers

Concept Paper

According to our experience and to a recent preliminary survey we conducted with the different stakeholders involved in drug development, there is no agreed upon understanding of the term "proof-of-concept".

In addition to the lack of consensus on the definition of this adaptable term, there is, in our opinion, the need to define what use has been made of this concept in the drug development area to date.

In the case of orphan drugs in particular, the demonstration of proof of concept can represent a green light to move forward to the next milestones: the passage from pre-clinical to first-in-man studies, the minimum amount of data or demonstrations necessary to convince decisions makers or investors at company level to start or continue investing financially or to convince regulators to grant an orphan drug designation, and later, a market authorisation.

Are these steps completed in a different manner and with a different level of proof of concept?

We can assume that because of their specificities such as the small size of the total patient population and the high level of innovation of some products, the required level of proof of concept could be different for orphan drugs. Is this really the case?

This 7th ERTC workshop will first aim to clarify the term “proof of concept” to hopefully reach a consensus on the language and thus improve the quality of communication between stakeholders. The analysis of the experiences at the COMP and CHMP level in the designation and marketing authorisation processes for orphan drugs will help acquire more accurate knowledge of how regulators evaluate the different levels of proof of concept presented by the sponsors and how this has an impact on the levels of evidence presented in the central marketing applications.

In the afternoon session, representatives of all stakeholders- industry, regulatory authorities, patients and academics- will present real-life cases of how the demonstration of the different proofs of concept are applied differently and explain how this impacts all aspects of their work.

A large amount of time will be left to the panellists and the attendees for an in-depth discussion on the complex puzzle created by this versatile term “proof-of-concept”.