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Orphandev, French Clinical Trials Network dedicated to Orphan drugs and therapeutics development for rare diseases

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Though rare diseases affect fewer patients than common diseases, developing drugs for them is subject to the same principles. But constructing and leading rare diseases clinical trials means also facing numerous difficulties which are more important in this field (methodology, little knowledge of the disease's evolution, choice of the judgment's criteria, small population's size, logistical matters...)

However during the last decade, France led several initiatives to improve the burden of rare diseases. Centres of Expertise were identified, ability centres have been appointed and a National Plan for rare diseases was developed.

Nevertheless, in spite of the great dynamic created by France, development and availability of orphan therapeutics remain problematic regarding rare diseases specificities. In this context, it is important to gather skills and strengths to make patients benefit from fundamental research's results and accelerate clinical trials.

Orphandev is a French Clinical Trials Network based on a strong collaboration principle with all actors involved (academics, industries and patients) dedicated to orphan drugs development. It was created by academics to help academics but also others actors involved in rare diseases' research. The Network' skills have already been dedicated to orphan drugs' trials: Charcot Marie Tooth (2004), Rett (2006) and Progeria (2008). Orphandev has intervened according to the mutualisation and translational research concepts from the experimental phase (in vitro and animals' tests) to the results' valorisation with every single actor involved in the study. With the experience gained and the successful results, we have developed an organisational concept in order to capitalise on the lessons learnt and optimise the trials process.

In a time of great therapeutics development with solutions coming from gene breakthroughs but not only, Orphandev allows for further improvement of the interface between fundamental research, clinical research and drug developments in rare diseases in a more operational way.

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