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From promising molecules to orphan drugs: Early clinical drug development

Marc Dooms
Center for Clinical Pharmacology, Belgium

Phase-1 (also known as "First-in-Man") clinical trials initiate the early clinical development of possible new medicines. By raising the dose of the investigational compound in healthy volunteers pharmacokinetic and –dynamic parameters are recorded alongside the safety profile of the new substance in humans. Patient participation in this early phase of clinical trials will be rather limited. After successful phase -1 trials, further phase -2 and phase -3 clinical trials in patients may lead to a marketing authorization. In the first 15 years of the European Union Orphan Drug Directive 4-5- percent of the orphan drug applications were authorizated. However, for many of these orphan drugs no phase -1 studies were required as these products were already well known pharmaceutical substances with a clearly defined pharmacological profile. Furthermore, for 19 orphan drugs, already authorized by the European Medicines Agency, the original rare indication was extended to another rare disease and no phase -1 trials were needed. For all the other orphan drugs clinical development started with regular phase -1 studies in human volunteers.

dooms@evonet.be