"Clinical trial design: Bayesian approach can be useful"?

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If prior information is available, the use of the Bayesian approach can offer important advantages in the design of clinical trials. For instance, it can increase the efficiency, i.e., result in a smaller sample size. In this presentation, two examples of clinical trials will be presented, in which the use of the Bayesian approach offered a feasible alternative to the classical frequentist design. The first trial was a non-inferiority study of a drug in children, which followed a study in adults. The use of the information from the adult study allowed to obtain a feasible design for the pediatric study. The other trial was a trial of immunotherapy in cancer patients. Data collected by a sponsor suggested a possibility of a differential treatment in subpopulations defined by levels of a biomarker. By using the Bayesian approach, a Phase II/III design was obtained that allowed to conduct a randomized Phase II trial to corroborate the previous finding and to seamlessly continue to Phase III in the subpopulation most likely to benefit from the treatment.