The role of the pharmaceutical industry in orphan drugs development

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The development of orphan drugs recorded a significant increase of interest from pharmaceutical companies in the last decades. This effect may be considered as an outcome of several events, which created a positive synergistic atmosphere. Main reasons can be considered the positive regulatory attitudes from both FDA and EMA, an interesting global market potential, the better understanding of the genetic factors responsible of most rare diseases, and finally the signiifcant support offered by patients' associations.

The largest database of orphan drug applications is the one from FDA: in the last 30 years pharmaceutical companies submitted almost 4000 requests for the orphan drug designation, with a success rate of about 70%. This is a large contribution, demostrating that, in the proper economic and regulatory scenarios, the pharmaceutical industry is willing to accept difficult challenges like the ones related to the development of orphan drugs.

Recent data demonstrate that these challenges are achieving important results: in the year 2014, the FDA approved 35 new drugs, and 15 of them were orphan drugs, the largest number in a single year since the regulation was in place. If you consider that the average time to develop a new drug is about ten years, it is interesting to underline that the pharmaceutical industry accepted these challenges at the turn of the millennium.