Researchers find how a drug for osteoporosis is effective to treat a rare disease

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Researchers at the Spanish National Research Council (CSIC) have found that the Bazedoxifene acetate, used to treat osteoporosis, is useful to treat a rare disease, the hereditary hemorrhagic telangiectasia, which causes bleedings and deteriorates the patient quality of life. The European Medicines Agency has granted an orphan designation to CSIC, which authorises to carry out clinical trials in order to commercialize it. An orphan drug treats low prevalence diseases (affecting 5 in 10.000 people) but has no investment from the pharmaceutical industry because it is not profitable.

The hereditary hemorrhagic telangiectasia (called HHT, or Osler-Weber-Rendu syndrome) is a rare disease affecting 2 in 10.000 people. "It is a genetic disease that causes nose bleedings, and red spots in the hands, face and mouth; and affects internal organs, with bleedings in the lungs, brain, liver and spinal cord", said the CSIC researcher María Luisa Botella, at the Biological Research Center (Centro de Investigaciones Biólogicas), who has led the research.

"It is not a fatal disease, but the symptoms lead to a reduction in a patient's quality of life. Due to the bleedings, the anemia and the necessity of blood transfusions are common", she adds.

Due to the low prevalence of the rare diseases and the special symptomatology, there are no therapeutical methods to treat them. Furthermore, in the pharmaceutical industry it is not profitable to invest in clinical trials for diseases of low prevalence. This is why the pharmaceutical industry called them "orphan".

There are two types of orphan drugs: the ones that are specifically developed to treat a rare disease, and those used in the treatment of a frequent disease that are also useful for a rare disease.

The designation is the result of the research led by the group of the Biological Research Center (Centro de Investigaciones Biológicas), at CSIC, and from the Hospital de Sierrallana (Cantabria). The Centre for Network Biomedical Research on Rare Diseases, the Asociación HHT España, and the Ministry of Economy and Competitiveness have contributed in the research.

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