

TWO-PRONGED APPROACH FOR THE TREATMENT OF POLYCYSTIC DISEASES

This invention refers to new compounds for the treatment of hepatorenal polycystic disease, and potentially different types of cancer. It concerns a new family of selective HDAC inhibitors (HDACis) derived from ursodeoxycholic acid (UDCA).

TYPE OF DEVELOPMENT

Drug.

DESCRIPTION

Histone deacetylases (HDACs) are a family of hydrolase enzymes that play a central role in the regulation of essential cellular processes such as differentiation, cell cycle regulation, modulation of cytoskeleton dynamics, metabolic processes, autophagy, and apoptosis, among many others. Alterations in the expression and/or activity of different HDACs have been described in a wide number of diseases including genetic disorders like hepatorenal polycystic disease, and several types of cancer. This invention refers to a new family of molecules that selectively inhibit different HDAC isoforms.

This invention concerns the description of a new family of selective HDAC inhibitors (HDACis) derived from ursodeoxycholic acid (UDCA), an endogenous and hepatoprotective bile acid. This family comprises several compounds with potent inhibitory capacity on different HDACs, including members highly selective for HDAC6 (cytoplasmic HDAC) and others displaying high selectivity for Class I HDACs (epigenetic HDACs).

The presence of UDCA in the structure of these compounds reduces the potential toxicity of drug metabolites and provides these compounds with hepatotropic features. These compounds have undergone extensive preclinical characterization for the treatment of hepatorenal polycystic disease (Caballero-Camino, F.J. et al, Hepatology, 2021). In addition, our current research clearly highlights the promising potential of these new drugs for the treatment of several types of cancer.

INDICATION

Patients with hepatorenal polycystic diseases.

Diseases in which HDACs are therapeutic targets (cholangiocarcinoma, colon cancer, melanoma, breast cancer, and multiple myeloma, among others).

NOVELTY/ADVANTAGE

- Highly selective inhibition.
- Reduced toxicity.



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Applicants:

Administration of the Autonomous Community of the Basque Country, The University of the Basque Country (UPV/EHU), University of Salamanca, and Bellvitge Biomedical Research Institute (Idibell).

COOPERATION GOAL

Company interested in the license and commercialisation of the product.

Collaboration for undertaking clinical trials.