ALLOPURINOL: A POTENTIAL NEW PHARMACOLOGICAL TREATMENT AGAINST AORTIC ANEURYSM IN MARFAN SYNDROME

A research group from Universitat de Barcelona and CIBER has identified a new therapeutic application for allopurinol with the potential to treat Marfan syndrome

The Need

Marfan syndrome is a rare genetic disorder that affects connective tissue and can lead to formation of aortic aneurysms.

Current pharmacological treatments are inefficient in preventing or blocking aneurysm progression. As a result, many patients eventually require preventive aortic surgery, a high-risk procedure associated with complications like stroke.

There remains an urgent unmet need for a safe, effective, and non-invasive therapy capable of preventing or significantly slowing aortic aneurysm development in Marfan patients.

The Solution

Allopurinol (ALO) is a well-known drug originally approved for reducing elevated uric acid levels.

In a preclinical study using a mouse model of aortic disease in Marfan syndrome, ALO demonstrated a remarkable capacity to block aneurysm formation and stop its progression.

The drug was effective in both preventive and palliative treatment regimens. Interestingly, its beneficial effect was not linked to changes in uric acid levels but rather to its antioxidant properties, which supports its potential repurposing for Marfan syndrome.

Innovative Aspects

- This proposal offers a novel repurposing of allopurinol, a low-cost, well-tolerated, and long-standing medication with an excellent safety profile.
- Unlike existing therapies, which have limited efficacy, ALO targets a new mechanism of action as a primary antioxidant, independent of its effect on uric acid.
- Future steps include determining the minimum effective dose and conducting a clinical trial in collaboration with clinical partners. The group is also exploring combination therapy with other drugs to assess additive or synergistic effects.
- Given its established use in clinical practice and potential cardiovascular benefits, ALO could offer a safe and accessible long-term option for Marfan patients, potentially transforming current treatment strategies.

Stage of Development: In vivo (mouse) preclinical validation of efficacy.

Intellectual Property:

- Orphan Drug Designation in 2025 (EU/3/25/3041)

Aims

Looking for partners interested in a license and/or collaboration agreement to develop and exploit this asset.

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