Technology offer IP-045

# New natural compound for the treatment of thalassemia and sideroblastic anemia

A new therapeutic approach based on sulforaphane, a natural compound derived from vegetables like broccoli, has been developed to inhibit the NLRP1 inflammasome, a key activator of inflammation and pyroptosis. By blocking this pathway, sulforaphane restores erythropoiesis in patient-derived cells. The technology shows promise for the prevention and treatment of thalassemia and sideroblastic anemia.

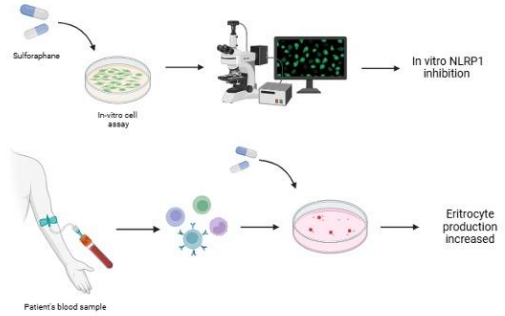


Figure. Overview of the experimental protocol

**State of development**

TRL-3 Proof of concept

**Objective of the collaboration**

License and/or co-development

**Industrial Property**

Spanish patent application

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## Market needs

Disorders like thalassemia and sideroblastic anemia result from disruptions in erythropoiesis, the process responsible for red blood cell production, leading to chronic anemia and impaired oxygen transport. In these diseases, dysregulation of iron metabolism or globin synthesis causes ineffective erythropoiesis and accumulation of abnormal erythroid precursors. Current treatments—including transfusions, chelation, bone marrow transplantation, and gene therapy—are often invasive, expensive, and not widely accessible. Despite recent advances, these remain rare diseases with limited therapeutic options. There is a clear need for safer, more cost-effective treatments that are also more convenient and less burdensome for patients.

## Technical solution from IMIB

The proposed technology is based on sulforaphane, a natural compound that inhibits the NLRP1 inflammasome, a key protein complex involved in triggering inflammation and pyroptosis. In *ex vivo* studies using primary cells from patients with thalassemia and sideroblastic anemia, sulforaphane restored erythropoiesis and improved red blood cell production. These results support its potential as a therapeutic agent for the treatment and prevention of these hematological disorders.

## Benefits

- Inhibition of the NLRP1 inflammasome, offering greater efficacy in modulating inflammation and restoring erythropoiesis compared to current therapies.
- Reduced need for blood transfusions and iron chelation, minimizing side effects and improving patient quality of life.
- Natural compound, already approved by the FDA and EMA for other indications, with potential for administration through various routes, facilitating formulation and clinical use.
- More cost-effective therapeutic alternative for rare diseases with currently limited treatment options.

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