

COMPOUNDS FOR THE USE IN THE TREATMENT OF ANEMIAS

A research group from CIBER, Universidad de Murcia and IMIB has identified several compounds as potential candidates for treatment of anemias

The Need

Anemias are **blood disorders** considered to be a cause of severe deterioration of quality of life, morbidity and decline in physical function and a risk factor for death.

Although there are several treatments for anemias, there is a **need of identifying new compounds** that can minimize the use of transfusions.

The Solution

Identification of compounds regulating and inhibiting a specific inflammasome pathway that is involved in the regulation of haematopoiesis.

Innovative Aspects

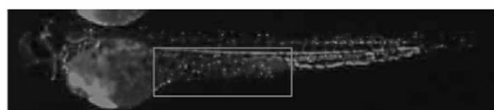
- Identification of different compounds that have in common the **regulation by inhibition of a specific metabolic pathway** involved in inflammation and tightly related to **haematopoiesis**.
- Potential compounds for treatment of rare anemias that have **no effective therapeutic options** yet.

Stage of Development:

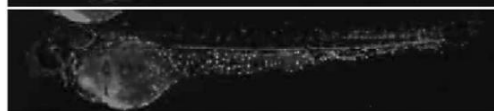
Pre-clinical assays have been performed with zebrafish models, cellular models and also in hematopoietic stem cells from Diamond-Blackfan anemia patients, with promising results.

Preparing the design of a clinical trial.

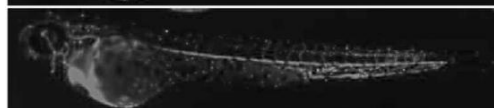
Control



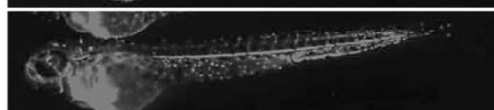
Compound 1
Dose 1



Compound 1
Dose 2



Compound 2
Dose 1



Compound 2
Dose 2

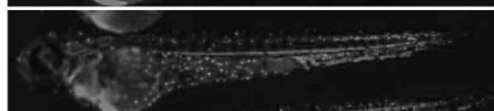


Fig 1. Significant increment of the number of erythrocytes in zebrafish model after treatment with identified compounds at increasing doses

Intellectual Property:

- PCT application.
- Entry into national phases.
- Orphan drug designation of a selected compound (EMA).

Aims

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

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