New method for analysing bone marrow populations by flow cytometry

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This project has received funding from the European Union's Horizon Europe Research and Innovation Programme 2021- 2027 under the Grant Agreement No 101080156

Congenital anemias are very complex disorders whose causes are largely unknown and whose treatments are non-existent or insufficient. The term Congenital Anemia englobes many rare diseases, which have in common a genetic defect that is usually hereditary and affects some part of the erythrocyte. NANEMIAR project sets out to develop a ground-breaking bone marrow-targeted mRNA formulation applicable to most non-iron anemias and provide proof of concept in β-thalassemia.
Bone marrow populations are characterized by their great heterogeneity, due to the variety of cell types and their different degrees of maturation. A precise identification of these diverse cell populations is crucial for the diagnosis of multiple hematological diseases, such as leukemias, lymphomas, anemias, and myelomas. In this study, we have developed a flow cytometry analysis method to accurately identify bone marrow populations. This technique will be employed to monitor the effectiveness of the NANEMIAR strategy in β-thalassemia mouse models and patients.

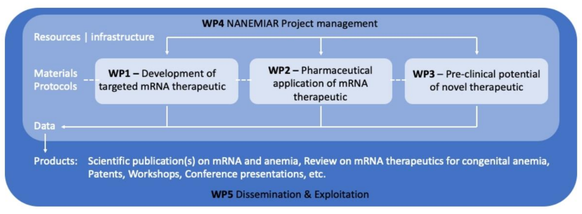
NANEMIAR

Ambition

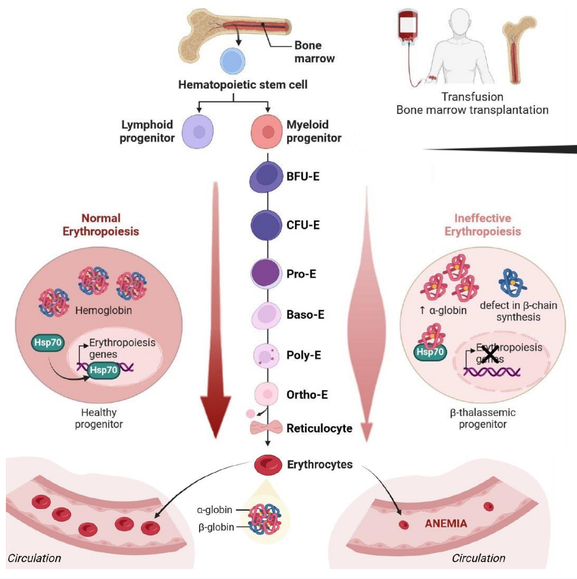
Development of an mRNA-based drug candidate for treatment of congenital anemias

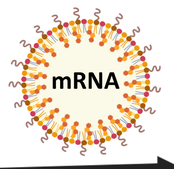
Specific objectives

- Development of therapeutic mRNA
- Pharmaceutical application of mRNA
- Pre-clinical potential of mRNA



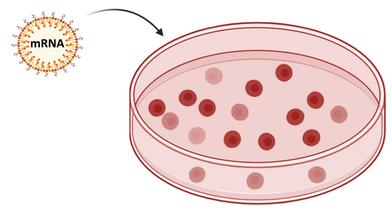
β-Thalassemia





LNP-targeted therapy

β-Thalassemia model



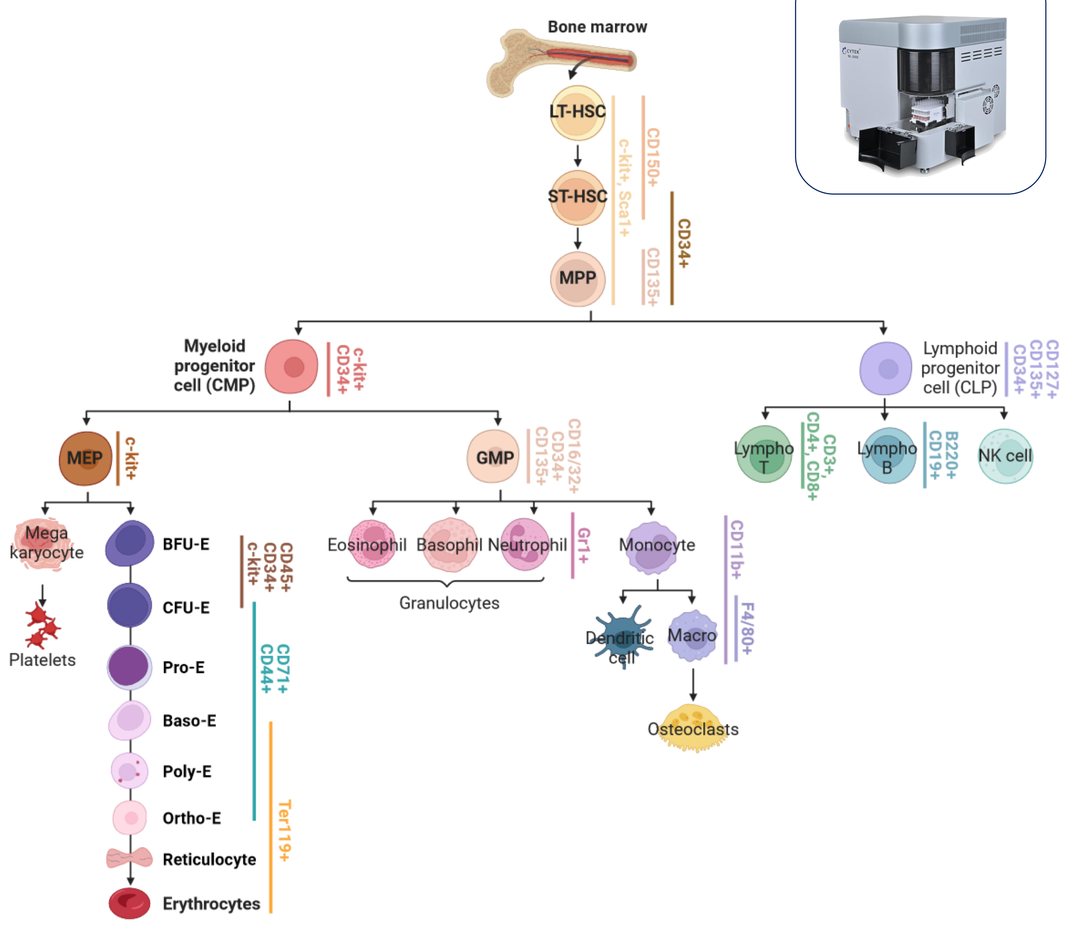
Myeloid progenitor cells from β-thalassemia patients

Study LNP-mRNA therapy impact on erythropoiesis

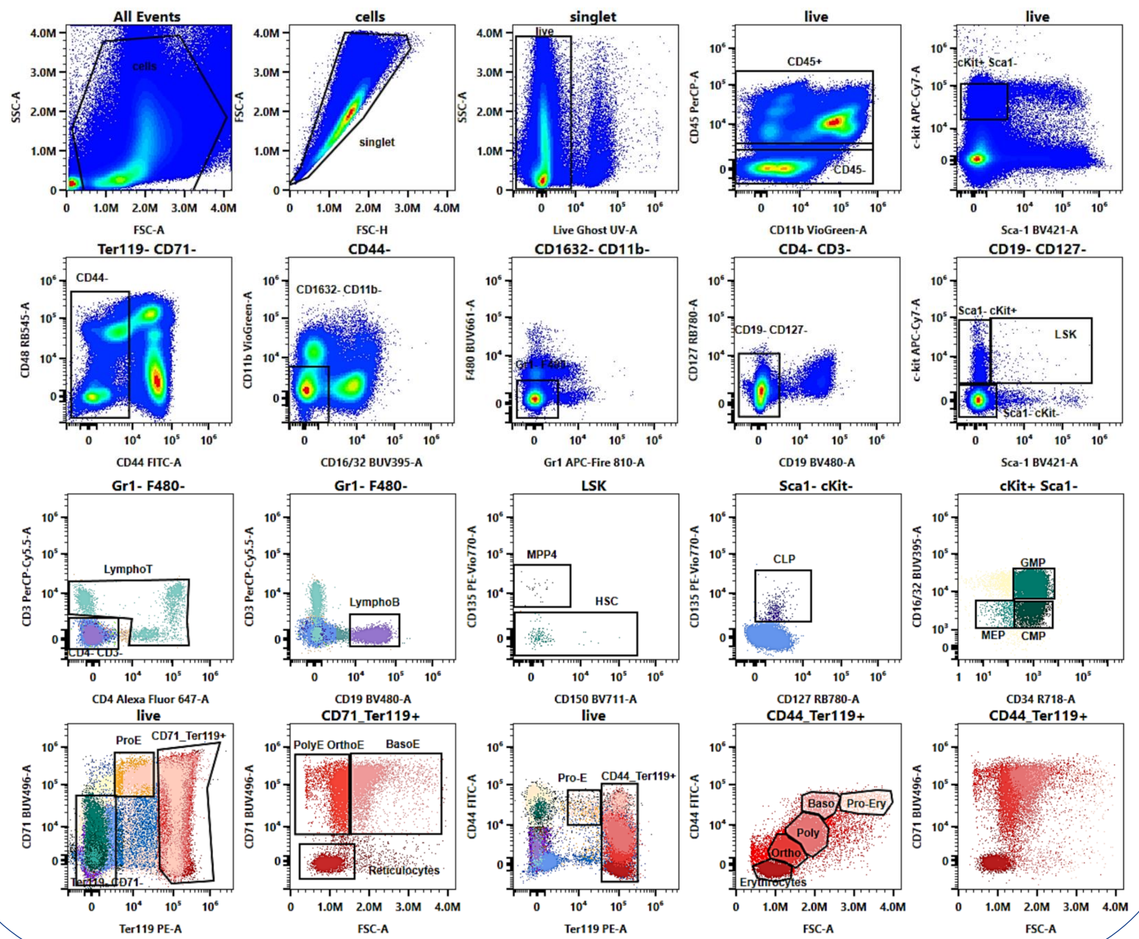
Optimised FACS analysis

Methods

FACS analysis



Results



Conclusions

The combination of the 22 selected hematopoietic markers allows differentiation of all cell populations present in the bone marrow and their degree of maturation. The possibility of combining all these markers in a single sample allows a complete analysis with a minimum amount of sample.

Applications

Flow cytometry is a simple and rapid method for the analysis of cell populations, which allows its use in patients with various congenital anemias to differentiate one pathology from another and to determine the degree of severity.
It could also be used to monitor the efficacy of treatments in different types of patients.

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