## NANEMIAR

#### Nanomedicine Approach to Normalize Erythrocyte Maturation in Congenital Anemia by Messenger RNA



Funded by the European Union

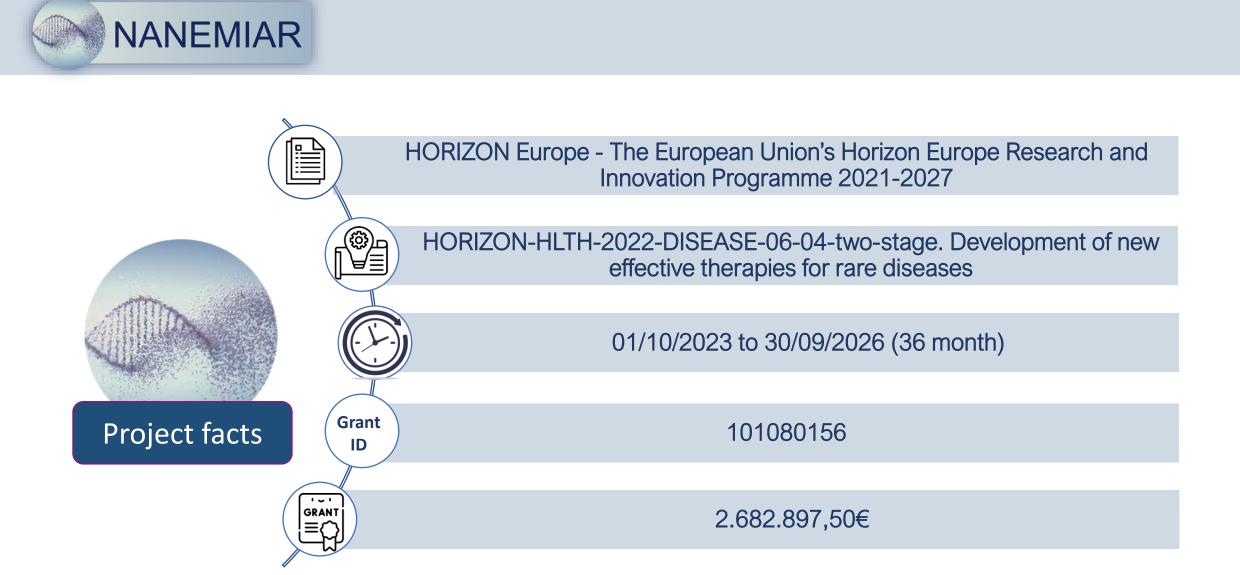






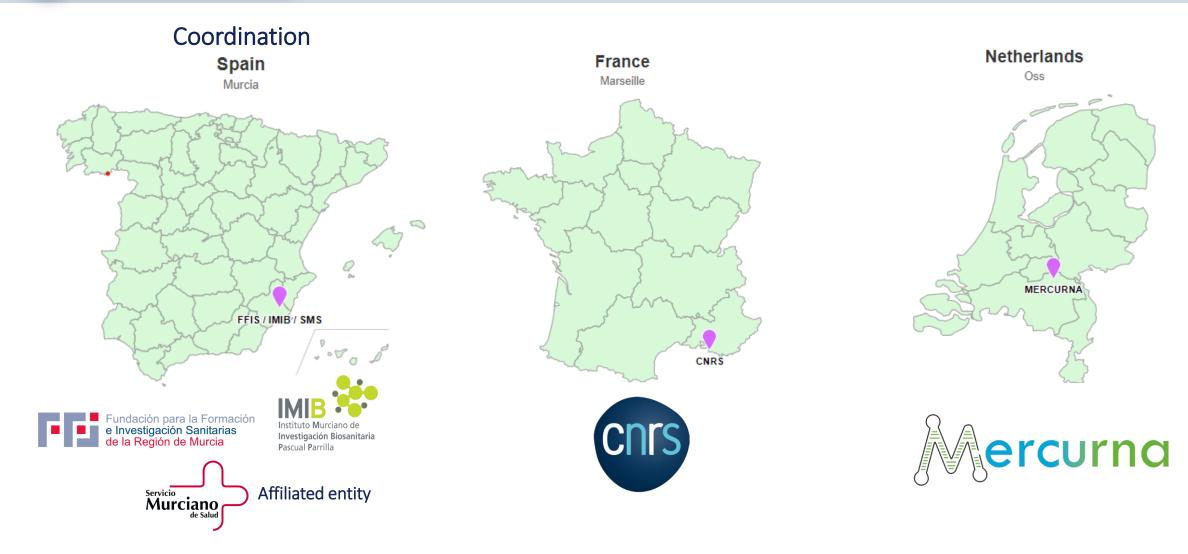








### NANEMIAR CONSORTIUM: 3 partners + 1 affiliated entity





#### NANEMIAR Kick-off meeting in Murcia, Spain (November 20, 2023)







### NANEMIAR Societal needs our project is addressing

Around 280 million people worldwide have a form of thalassemia (1 in 10.000 in the EU)<sup>1</sup>

Current treatment (small molecules, blood transfusion) is insufficiently effective in duration and associated with side effects

€15,000 per patient per year => Estimated saving of €5-6B compared to gene therapy approaches

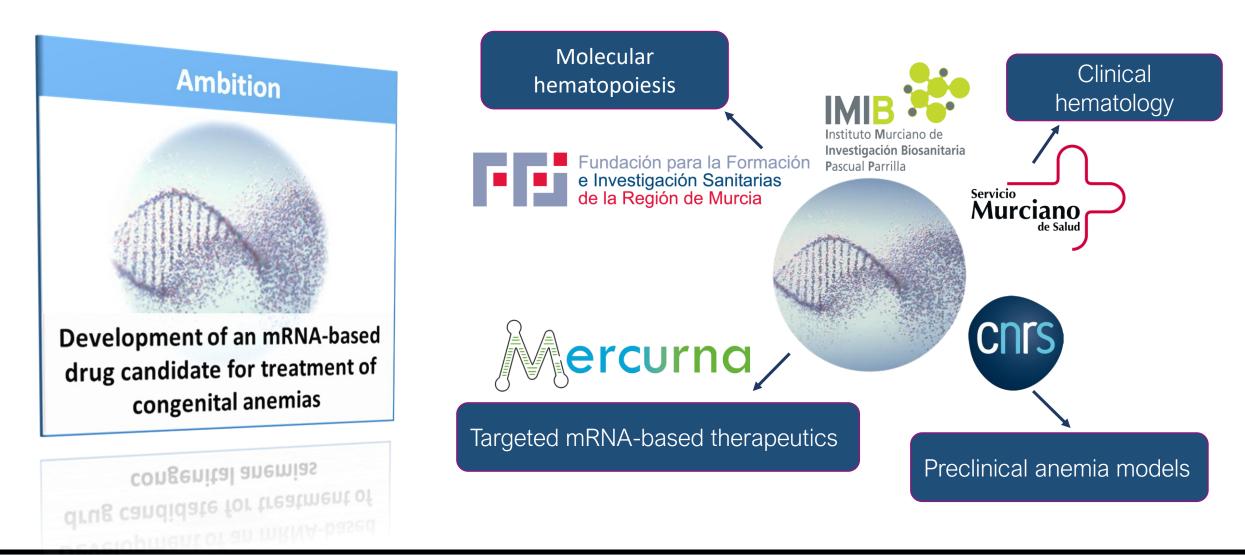
Kattamis A, Forni GL, Aydinok Y, Viprakasit V (2020) Changing patterns in the epidemiology of beta-thalassemia. Eur J Haematol 105: 692-703



β-thalassemia

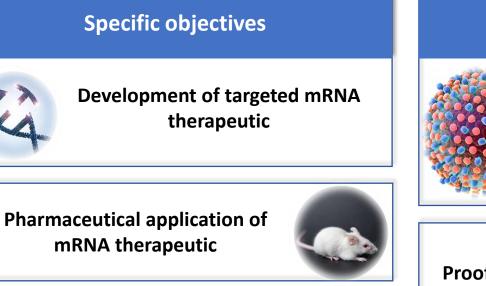
1.

### NANEMIAR Ambition of NANEMIAR





### NANEMIAR Specific objectives and outcomes



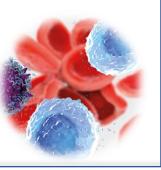
Pre-clinical potential of novel therapeutic

 $\begin{array}{l} \mbox{Proof-of-concept for a safe} \\ \mbox{treatment option for } \beta \mbox{-} \\ \mbox{thalassemia} \end{array}$ 

**Outcomes of the call** 

A nanomedicine base-

system for next-generation gene therapies



Combining expertise from academic, clinical and industry partners in Spain, France and The Netherlands, we aim to generate an innovative treatment option for congenital anemia, and advance knowledge in targeted therapeutics.



#### **NANEMIAR** Expected impact

#### Expected impact FU



"Health burden of diseases in the EU and worldwide is reduced through effective disease management"

Aligning with The Horizon Europe Strategic Plan for improved therapies and rare diseases.







#### **Scientific Impact**

New breakthrough scientific knowledge on mRNA therapies

Strengthtening R&I on congenial anemia

Promoting knowledge transfer and Open Science

#### **Societal Impact**

Addressing needs for new therapeutic options that ultimately increase patients' ability to work/participate in society

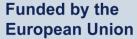
Strengthtening awarenss and uptake of mRNA appraches in society

#### **Economic Impact**

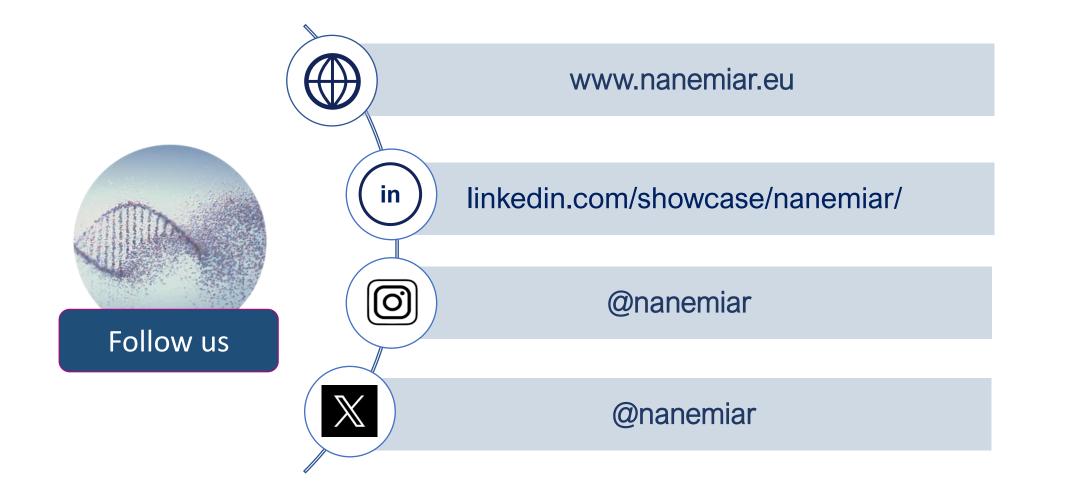
Promoting innovation-based growth in academia and industry

Leveraging R&I investment with potential for reduced development and healthcare costs











## NANEMIAR

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