# miRNA-based strategy to expand cell therapy potential for treating diabetes

MIT linQ IDEA<sup>2</sup> Global 2019 20-21 June

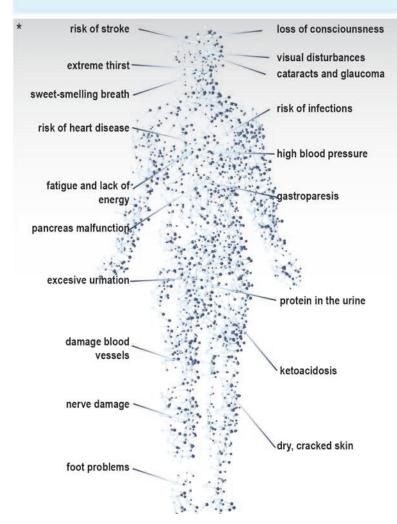


María Salazar Roa Spanish National Cancer Research Center

### Type 1 Diabetes



Type 1 DIABETES (T1D) is an autoimmune disease that destroys the beta cells, leading to high blood glucose levels and therefore, metabolic failure.\*\*



#### SOCIAL AND ECONOMIC COSTS

insulin injections constant glucose control



90,000 children diagnosed each year.\*\*

>500,000 children are living with this disease globally\*\*

healthy diet and lifestyle



people diagnosed in 2018

14.9 billion USD only in US

By 2025, there will be 12,3 million

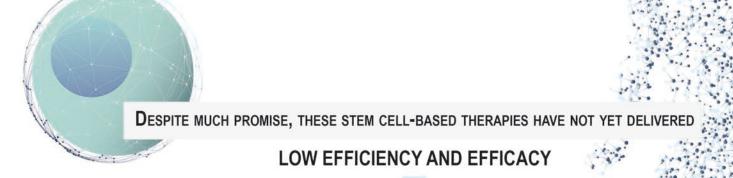
– with the annual growth rate
higher in developed countries
(Spain leading this list)\*\*

<sup>\*</sup> WHO reports https://www.who.int/news-room/fact-sheets/detail/diabetes

<sup>\*\*</sup> Katsarou, A. et al. (2017) Nat. Rev. Dis. Primers doi:10.1038/nrdp.2017.16

### Existing strategies and their limitations

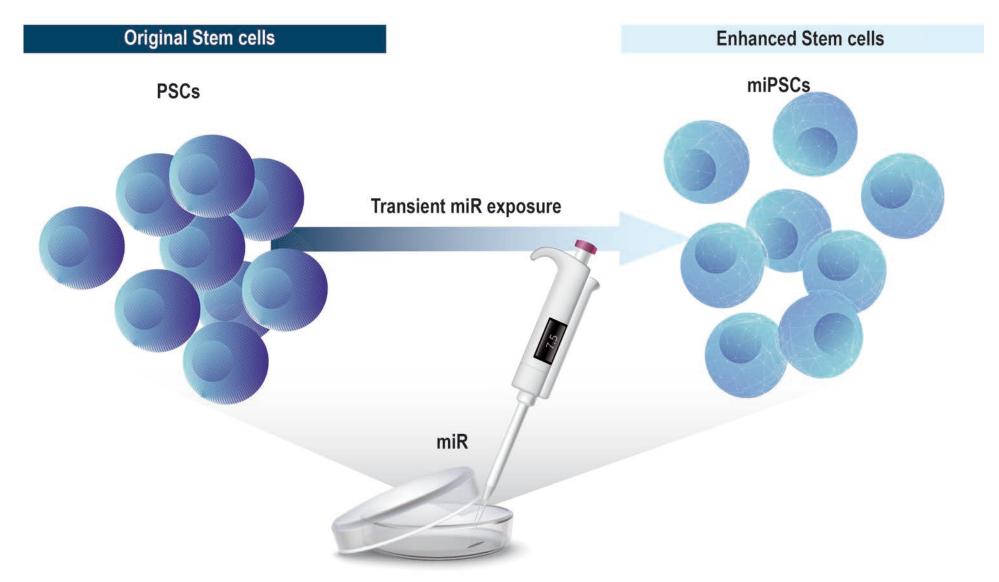




There is a need to improve the current stem cell-based technology so it can be considered fully as a clinical tool

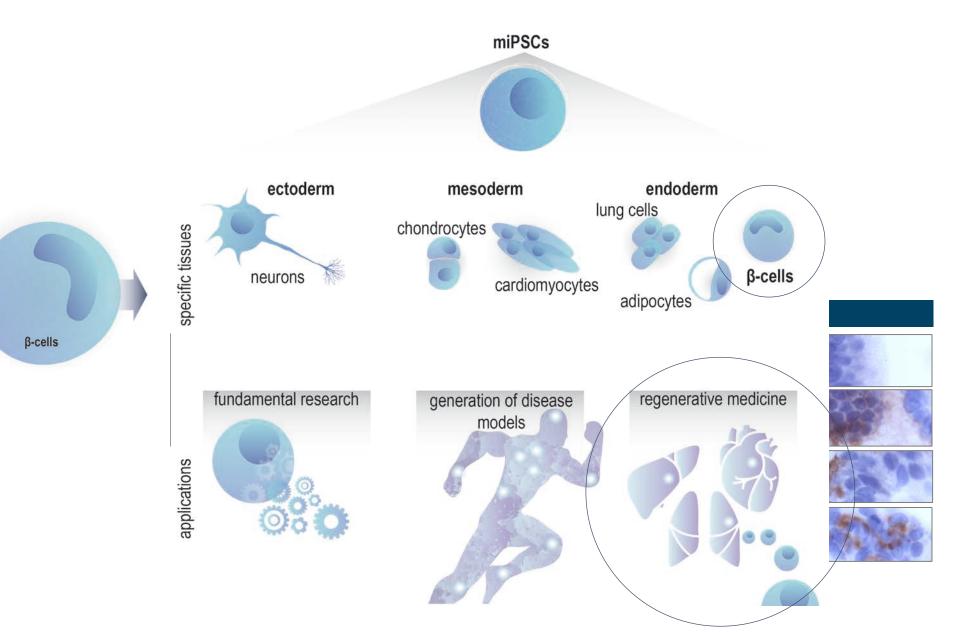
# A breakthrough strategy: **the method**





# A breakthrough strategy: the platform





germline transmission & differentiation efficiency

DNA methylation dynamics, in a reversible way: permissive for a proper differentiation.

ACHIEVED GOALS

miR potentiates differentiation of PSCs

chimera

contribution

miR in specific differentiation scenarios

patent application WO 2018/215662 A1

#### Social and economic Impact and Responsible Innovation



#### to an applicable and efficient therapy



getting closer to the **definitive cure** of the disease







Scientific Research



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