

### ***(Impact statement)***

*Type I diabetes is a devastating autoimmune disease, with no cure, now managed by lifelong insulin injections. Although promising, Regenerative Therapies are still showing a number of limitations and therefore, narrow success on this disease. Using our novel microRNA-based technology, we offer **MlmoPancreas**: an encouraging solution to those issues, that will revolutionize the current state-of-the-art for pancreatic cell replacement.*

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### **Type I diabetes is an unmet need**

Type I diabetes is a devastating autoimmune disease, with no cure, now managed by lifelong insulin injections and constant glucose control. It has a huge social and economic impact, affecting not only to the patients and their families, but extremely to governments and healthcare systems.

The expected growth of market on T1D is about 4% in the next few years.

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### **THE PROBLEM: regenerative therapies in T1D are the “holy grail”, but still show limited success**

The biotech industry has seen this opportunity and is striving to develop new treatments to chase the holy grail: a cure.

**The cell therapy on T1D is at its peak.** Around ten companies are currently developing the technology to make possible the needle-free revolution.

But the reality is that such technology is still far from the market:

- Current competitive market is trying to develop the technology to: (i) obtain de stem cells, (ESCs, iPSCs); (ii) differentiate them to pancreatic cells; (iii) include them in a particular device that allows free traffic of glucose/insulin but does not permit the entry of immune cells (thus avoiding the immune attack). Those ten companies are developing such strategy, at different levels.
  - Some of those companies are devoted to improve the cells and some others, to improve the encapsulation devices.
  - However, **the success is still limited by the insufficient potential of stem cells to (i) survive under challenging conditions; (ii) differentiate and mature to be functional; (iii) maintain those properties in the long-term.**
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### **OUR PRODUCT: MlmoPancreas capable of producing insulin**

Our technology will overcome the current issues. We offer a suitable methodology to significantly improve the **stem potential** of the initial material, **their survival and their capacity to specialize** into pancreatic **mature and functional** cells.

We are intended to be **not only better but different** from our competitors: our novel product, **MlmoPancreas**, is much more than a cluster of cells, but a self-organized tridimensional structure that accomplishes different pancreatic cell types together, working together for a more efficient outcome. Interestingly enough, **MlmoPancreas** are created **from pancreatic progenitors** (avoiding concerns about pluripotency of ESCs/iPSCs).

- miR-based technology makes them mature, functional and stable.
- **Our vision: Long-life restoration of blood glucose control.**

## **THE MECHANISM OF ACTION of our miR, deeply studied during the last 5 years on stem cells**

Our miR-based technology has been successfully tested (i) in multiple applications and (ii) by several collaborators all over the world for several years.

The mechanism of action is epigenetic. The miRNA erases transiently and reversibly the DNA methylation, in a manageable way, to promote better differentiation outcomes afterwards.

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## **OUR TECHNOLOGY : MImoPancreas for cell replacement**

(brief description of the scheme)

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## **Our market strategy: making STRATEGIC ALLIANCES**

The companies **devoted to improve the encapsulation will be potential partners** to approach. Together, **we will establish strategic alliances** to efficiently compete with the rest of the market.

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## **OUR TEAM – stem cell biology frontier research**

**Our team has a fully-committed project manager, who has developed the whole science behind the idea and will lead the upcoming startup. The complementary team members-necessary to cover all the needs of the startup- will be recruited throughout the next months.**

- **María Salazar-Roa**, project manager. Staff scientist at CNIO. More than 6 years of experience in stem cell biology, cell proliferation and differentiation. 37 articles (9 as first author, 2 as corresponding author); more than 8.000 citations; one patent directly related to the asset; awarded by MIT (Boston, US) and Caixa (Barcelona, Spain) to participate in *IDEA2 Global 2019* and *CaixaImpulse 2019* valorization and mentorship programs.

**She counts with the support of the following team:**

- **Carolina Pola**, as Director of Innovation at CNIO.
  - **Marcos Malumbres**, scientific advisor. Head of Cell Division and Cancer lab at CNIO.
  - **Nuria García** (full-time PhD student) and **Aicha EIBakkali** (part-time technician) will participate actively in the project.
  - Our advisory board (**Petra Krauledat**, **Peter Bryant**, **José Luis Cabero**, **Sotirios Karathanasis**) mentors and monitors periodically our progress.
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*Our mission is making the cure possible*

**(Headline)**

**MImoPancreas** will revolutionize the current state-of-the-art for pancreatic cell replacement. It is an encouraging solution to make possible the regenerative medicine in T1D.