

## Impact “Mad Lib”: miR-Based therapy

For type 1 diabetes patients, their families, caregivers, healthcare professionals in the first place- but also for healthcare facilities, hospitals, clinics, healthcare systems and health insurances companies-

**it's a challenge to** afford this disease, which currently has no cure and affects more that 415 million people allover the world, costs more that 600 billion USD and causes 5 million deaths per year.

**Today, their best option is** controlling disease progression, by insulin injections and maintenance of healthy diet and lifestyle. Of importance, cell-based therapies were well positioned as a potential cure of the disease. Indeed, islet transplantation procedures have been performed worldwide but with low success, given the lifelong need for immunosuppression required and the observed time-limited functionality of the transplanted tissues. In turn, stem cell-based therapy has emerged as an encouraging alternative, based on the use of reprogrammed cells derived from the diabetic patient, avoiding the need of immunosuppression. The field of stem cells therapeutics is rapidly evolving, with several ongoing clinical trials exploring its effectiveness.

**However, because of** the intrinsic difficulties to obtain gold-pluripotent cells by standard reprogramming, this therapy

**yields** to poor-quality pluripotent cells, loss of pluripotent state, poor differentiation potential and lack of maturation properties (functionality of beta cells generated).

**Thus, there is a need of** finding an improved methodology to easily generate high-quality pluripotent cells from patients, capable of differentiating to pancreatic beta cells, mature and functional, long-term producers of insulin once they are implanted back into the patients.

**If solved, it would have the impact of** revolutionizing the cell-based therapy for type 1 diabetes and the socio-economic repercussion of this methodology would be undeniable enormous: not only for patients, their families and healthcare professionals benefited in the first place, but also for the global Healthcare System, Heath Insurances, Governments worldwide...who are struggling to meet the cost of diabetes care.

**Solving this need can be achieved by** using our breakthrough methodology: a brief exposure to a single molecule (a microRNA) makes any stem cell tested so far (independently of its origin) more prone to efficiently differentiate into the desired specialized tissue: in this case, pancreatic beta cells, mature and producers of insulin,

**and will be demonstrated/proven by** analyzing parameters such as dependency on immunosuppression throughout time, dependence on insulin injections, suffering of secondary effects of diabetes progression... in diabetic patients transplanted with our *patient-derived proficient beta cells*, compared to the existing stem cell-based therapies.