

ENDOCELLS



Innovative Medicines Initiative

# Towards a cure for diabetes

Improving beta-cell function and  
identification of diagnostic biomarkers  
for treatment monitoring of diabetes  
(IMIDIA)



- **Diabetes** is a chronic disease that afflicts 200 million people in the world. For various reasons the prevalence of this disease is increasing and the projections are impressive.
- In 2050 20% of the population worldwide will have some type of glucose regulation dysfunction. To understand the mechanism of the disease, to prevent and to treat diabetes is one of the major medical challenges of this century.
- Type 1 diabetes results from autoimmune destruction of Beta cells (the cells which produce and secrete insulin), while type 2 diabetes is caused by a combination of insulin resistance and inadequate insulin secretion.
- Thus, in both type 1 and type 2 diabetes, the functional Beta cell mass is not sufficient to control blood glucose concentration.
- Regenerative medicine is one of the research areas where possible new treatment could be discovered.

- **French Biotechnology company funded in S2 2004 in Paris.**
- **A Team:**
- **Three scientists/ inventors:**  
Prof. P. Czernichow, Dr R. Scharfmann and Dr Ph. Ravassard.
- **Business Angels**
- **A collaboration with INSERM and CNRS**
- **A President, D. Slagel and a CEO, A-F Weitsch.**

- **Endocells** is dedicated to the generation and development of various human cell lines with endocrine properties.
- Initial and current focus :**diabetes**.
- **Generation of human beta cell lines:**
  - to be used for both basic and applied research
  - to be applied as replacement cell therapy in chronic diseases
  - to be used as a source of substrates for early detection of antibodies for the development of diagnostics.

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- Patents: three major technology patents.
  - Deposition of MCBs at the Pasteur Institut.
  - Collaborations with European pharmaceutical and biotechnology companies.
  - Collaborations with academic institutions.
  - Partner of IMIDIA and another FP7 program.
  - A first product EndoC-betaH1 reaching the market Q4 2011.

## EndoC-betaH1:

- A tool product generated and developed by Endocells to be validated by large Pharma within IMIDIA .
- A product to be used as research and knowledge tool by corporations and academics
- A product to be used to discover and develop novel drugs (Biologicals as well as NCEs) to treat /prevent the development of diabetes.

## IMIDIA :

- A program dedicated to the development of “Islet cell Research”.
- **22 participants:**  
European pharmaceutical companies, European academic Institutions and 1 SME (Endocells).
- **Start:** Early 2010
- **Progresses:** on target.

## IMIDIA-ENDOCELLS

- Use of Endocells' beta cell lines to better understand beta cell function and beta cell development.
- Close collaboration with academics and pharmaceutical companies.
- Pharmaceutical companies are validating the robustness of Endocells' human beta cell lines.
- Generation of breakthrough knowledge on human beta cell function toward a cure for diabetes.





## IMIDIA-ENDOCELLS

- Sharing of critical scientific knowledge for the future development of new therapeutics.
- Access to major expertise in the field of diabetes.
- Combination of various technologies to move toward the understanding of diabetes.
- Financial contribution.
- A publication: “A genetically engineered human pancreatic beta cell line exhibiting glucose-inducible insulin secretion”. (J. Clin. Invest. Sept. 2011)

- **Regenerative Medicine** focuses on the creation of living tissues / cells to replace damage tissues and restore normal function.
- For type 1 diabetes these cells are the Insulin producing cells of the endocrine pancreas : the Beta cells.
- These cells should have 3 major properties:
  - ✓ Unlimited source, **obtained by Endocells**
  - ✓ « Key functions » close to normal beta cell, **obtained by Endocells**
  - ✓ Excellent safety profile, **on going**