A French Biotechnology company dedicated to the development and production of human endocrine cell lines

Innovation In Healthcare
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French Biotechnology company

- Founded in S2 2004 in Paris by a team of
- Three scientists/inventors:
  - Prof. P. Czernichow, Dr R. Scharffmann and Dr Ph. Ravassard.

- Business Angels
- A collaboration with INSERM and CNRS
- An Institutional Venture Fund
- In 2006 joined by a CEO, Dr. A-F Weitsch.
Technology and Innovation Developments

• Generation and development of sustainable source 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 (drug discovery and toxicology testing)
  ➢ 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.
Intellectual property

- Two US patent applications and one PCT application in its national phases (WO 03/033685 A2) related to the method of producing human beta cell lines. Patent granted in the UK and New Zealand and in Europe. Exclusive world-wide license.

- One PCT (WO 2008/102000) filed by Endocells in February 2008 with priority date 02.2007. Covers the validation of technologies for the creation and development of reversibly immortalised human beta cell lines, its use in diagnostics, in therapeutics and as a discovery tool.

- A new patent filing in October 2011 (US provisional) to protect technologies for the creation of third generation human beta cell lines.

- Material protection of human cell lines. Deposition of Master Cell Banks (CNCM, Institute Pasteur).
Innovative Medicine Project IMIDIA*:

### IMPROVING BETA-CELL FUNCTION AND IDENTIFICATION OF DIAGNOSTIC BIOMARKERS FOR TREATMENT MONITORING IN DIABETES

**8 Pharma Companies:**
- Sanofi (Coordinator)
- Servier (Deputy Coordinator)
- AstraZeneca
- Boehringer Ingelheim
- Eli Lilly Ltd
- Novartis
- Novo Nordisk
- F. Hoffmann-La Roche

**13 Academic Partners & Biotech:**
- Université de Lausanne
- Centre National de la Recherche Scientifique (CNRS)
- Commissariat à l' Energie Atomique
- Imperial College of Science, Technology and Medicine
- Institut Suisse de Bioinformatique
- Institut National de la Santé et de la Recherche Medicale (INSERM)
- Medizinische Hochschule Hannover
- Technische Universität Dresden
- Universita di Pisa
- Université Paris Diderot
- Université de Geneve
- Vrije Universiteit Brussel
- Endocells SARL

**FINANCING:**
- IMI funding: € 7.074.760
- Other contributions: € 3.750.920
- Pharma EU costs: € 15.081.800

**TOTAL PROJECT COST:** € 25.907.480

**STARTING DATE:** 1.2.2010

**DURATION:** 60 months

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IMIDIA: General goals

• Novel tools for the study of:
  – Human β-cell development, function and survival
  – Human β-cell functional modulation by potential therapeutic compounds
  – In-vivo β-cell imaging

• Biomarkers:
  – For the diagnosis and prognosis of β-cell failure
  – For monitoring diabetes progression and treatment

• Knowledge:
  – Of novel pathways and sites that control β-cell proliferation, differentiation and apoptosis,
  – Of the role of nutrient-regulated pathways in controlling β-cell mass and function
• Access to major expertise in the field of diabetes (innovation & application).
• Close collaboration with academics and pharmaceutical companies.
• Pharmaceutical companies are validating the applicability and robustness of Endocells’ human beta cell lines.
• Use of this beta cell lines to better understanding of beta cell function and development.
• Generation of breakthrough knowledge on human beta cell function toward a cure for diabetes.
• Sharing of critical scientific knowledge for the future development of new therapeutics.
Achievements-to-date

- Established a robust technology platform which will be used to develop other human endocrine cell lines.

- Generated tumoral and non-tumoral human beta cell lines which closely resemble human primary pancreatic beta cells. **World first.**

- Licensing out its first product EndoC-BH1.

- Developed insulinoma to be used for the development of diagnostic kits, in partnership with a diagnostic company.

- Creation of a world-wide recognised scientific team (Founders of the company).

- Creation of a management team with strong expertise in business development, pharmaceutical and biotechnology business.
Ethical issues

- Human material issued from voluntary interrupted pregnancy
- Authorisation obtained from ABM.
- Anonymous written inform consent executed by the women.
- Protection of confidentiality of personal information.
- No financial gain for the women or the hospital.
- Full traceability of the material.
- Work is performed under existing laws and regulation applicable in France.