Juvenile Diabetes Cure Alliance
The Voice of the Donor For a Cure

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Aaron Gorin
Director of Research Analysis
212.308.7433
apg@thejdca.org

JDCA Visit to the Diabetes Research Institute

What's New: This week we visited the offices of the Diabetes Research Institute (DRI) in Miami, Florida. The purpose of the trip was to learn about progress and next steps of key initiatives of the DRI's BioHub.

Background: The BioHub is a research initiative with a stated goal of curing type 1 diabetes. Specifically, the DRI is pursuing a "biological cure" that would replace the insulin-producing islet cells through transplantation, by simultaneously pursuing three interconnected research pathways: 1) optimal site selection for the transplanted cells; 2) islet cell protection; and 3) islet cell supply. In previous reports we have noted that many of the BioHub projects have potential to result in a Practical Cure.

Most Progress: Two areas within the BioHub project have progressed enough to be slated for human trials in 2014:

1. *The scaffolding project.* Human trials targeted to start in the back half of 2014. The scaffold utilizes an innovative material that will not be rejected by the body and holds several hundred thousand islets cells. Enough test units of the scaffold have been produced to field a study of approximately 200 people. The DRI is preparing primary and backup options to move the scaffold project into human trials during 2014. The three options concurrently being perused are: United States, Canada, and China. The final testing location has not yet been selected.
2. *Regulatory T Cells.* Regulatory T cells are sourced from bone marrow and are may have the capacity to induce immune tolerance – cell protection - by suppressing the autoimmune response. DRI is preparing to conduct a multi-center clinical trial in 2014, exact timing TBD.

Other Biohub Initiatives: A few additional research projects are worth noting even though none of them have projected human trial timeframes.

- *The venuous sack project.* This project utilizes a vein as the location for inserting islet cells. This is being developed primarily by DRI collaborators and time to human trials is not yet projected. That said, it is worth noting that preferred insertion site seems to be shifting to the omentum rather than a vein.
- *Biologic thrombin,* a new drug solution that will prevent the autoimmune attack. The DRI hoped to piggyback this new drug with the scaffold trials in 2014 but the FDA is requiring an Investigative New Drug application which will push the trial timing beyond this year.
- *Cell supply.* A large supply of insulin-producing beta cells for transplantation is key to a full solution. This research stream, which primarily involves retraining stem cells, does not yet have a target timetable to transition from bench to animal testing and human trials.

Key Takeaways: The DRI is moving forward with the BioHub as envisioned a year ago. One good outcome is that the Scaffold project and a Regulatory T Cell initiative seem likely to enter human trials during 2014, mainly because the DRI has been innovative and entrepreneurial in identifying primary and secondary testing locations. Concurrently, other key BioHub initiatives have not made enough progress to move into trials during 2014.

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