JDRF Three-Year Research Goals

JDRF Juvenile Diabetes Research Foundation International
dedicated to finding a cure

Regeneration Program:

1. Identify the key cellular target(s) for therapy
2. Discover and validate at least one biochemical pathway
3. Discover and validate at least one potential drug target
4. Discover at least one small molecule or biological factor for lead optimization
5. Decision on clinical viability of Gastrin combination therapeutics
6. Develop Biomarkers

Replacement Program:

1. Proof of concept of human embryonic stem cell-derived beta cells in diabetic animal models
2. Identification, characterization, isolation, and expansion of a pancreatic beta cell precursor
3. Proof of concept of non-pancreatic, non-embryonic stem cell-derived glucose-responsive, insulin secreting cells in diabetic animal models
4. Go/no-go decision for clinical trials of xenotransplantation
5. Achieve proof of concept for clinical utility of islet encapsulation technology in non-human primates

Autoimmunity Program:

1. Complete enrollment for clinical trials that reposition 4 FDA-approved immunotherapeutics
2. Identify/validate at least 1 new beta cell target for antigen-specific immunotherapeutics
3. Complete preclinical evaluation of at least 1 novel technology for tolerogenic delivery of immunotherapeutics
4. Launch cell based therapeutic trial(s) in new onset type 1 diabetes
5. Clinical evaluation of insulin-based immunotherapeutics
6. Application of existing and new knowledge of the immunopathogenesis of human type 1 diabetes for disease reversal and prevention
7. Identify at least 1 biomarker of immunologic efficacy
8. Completion of enrollment for Anti-CD3 Phase III clinical trials

Complications Program:

Trans-Complications

1. Clinical: Complete enrollment for 2 clinical trials with marketed drugs and begin clinical trial for 1 new drug
2. Preclinical: Validate 3 targets for trans-complications

Diabetic Retinopathy

1. Clinical: Evaluate drug efficacy in macular edema and in retinopathy
2. Preclinical: Validate 2 targets for diabetic eye disease

Diabetic Myocardial and Vascular Complication

1. Preclinical: Validate 3 targets of myocardial or vascular complications

Diabetic Nephropathy

1. Clinical: Evaluate safety and efficacy of 2 drug candidates in early stage clinical development
2. Preclinical: Validate 2 targets; Identify promising surrogate clinical endpoint

Diabetic Neuropathy

1. Clinical: Test surrogate endpoint for peripheral neuropathy
2. Preclinical: Validate 2 targets for peripheral or autonomic neuropathy

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Metabolic Control Program:

1. Complete JDRF Continuous Glucose Sensor Trial
2. Launch and enroll home trials of automated insulin pumps – (first generation artificial pancreas prototype)
3. Complete enrollment for proof of concept human drug trial to treat beta cell metabolic stress
4. Design and launch proof of concept human drug trial to prevent hypoglycemia

Accelerators:

Biomarkers

1. C-peptide established as a primary endpoint for clinical trials
2. Discover and validate new biomarkers for immunoregulation and diabetes complication therapeutic interventions

Imaging

1. Achieve non-invasive techniques for measuring beta cell mass, beta cell regeneration, functionality, and islet inflammation

Physician and Patient Outreach/Patient Recruitment

1. Improve provider acceptance of continuous glucose sensor and an artificial pancreas
2. Go/no-go decision on patient registry
3. Develop effective approaches to facilitate clinical trial recruitment

Regulatory Pathway/Advocacy

1. Provide data for accelerated decision making

Bioinformatics

1. Develop effective tools for data analysis and knowledge discovery