Diabetes: Targeting Safe and Effective Prevention and Treatment

Advocacy Perspective

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Chief Scientific Officer

JDRF Juvenile Diabetes Research Foundation International

dedicated to finding a cure

JDRF Mission

To find a cure for type 1 diabetes and its complications through the support of research

JDRF Research Goals

- Restoration and maintenance of normal blood sugar
- Prevention and treatment of complications
- Prevention of diabetes and its recurrence

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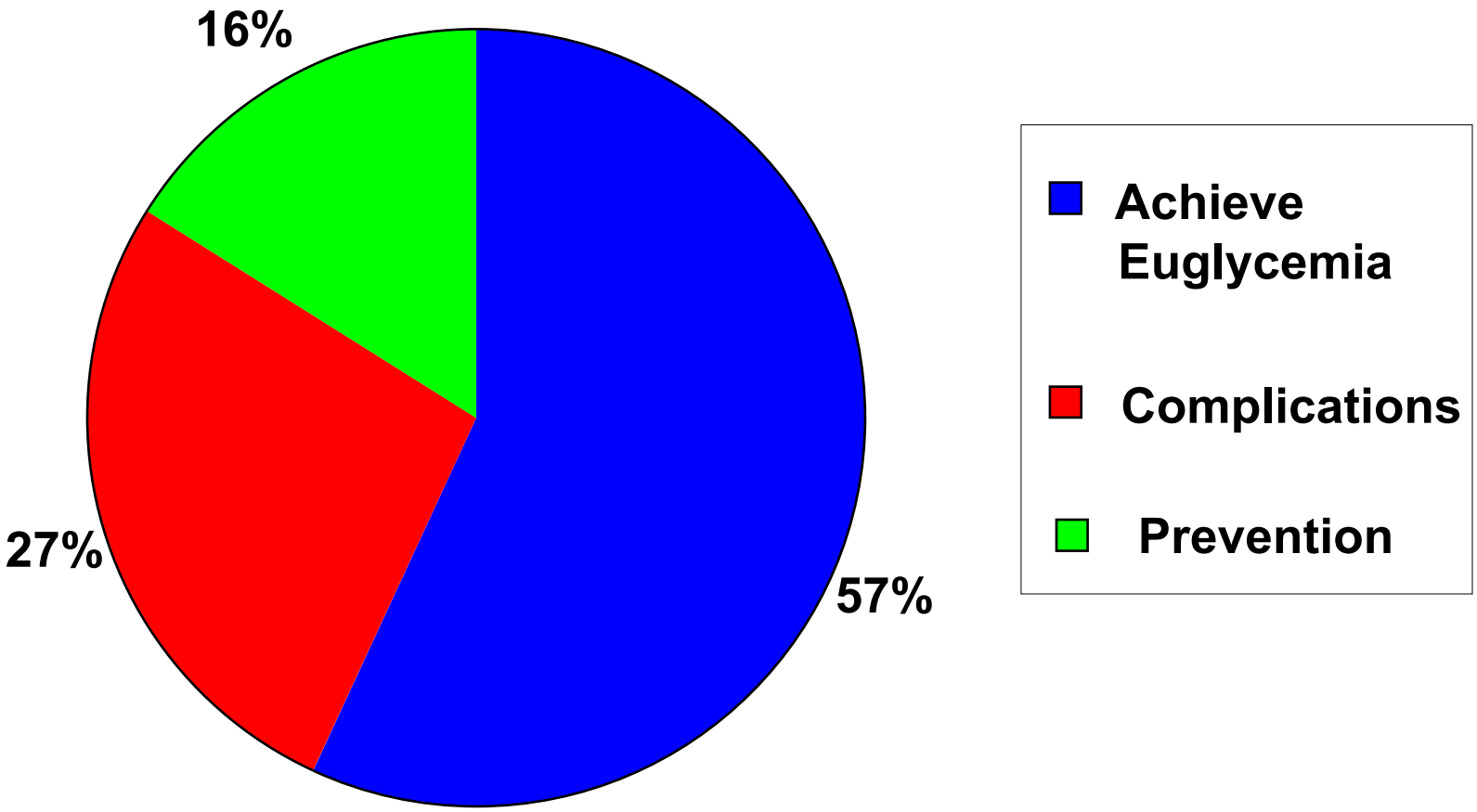
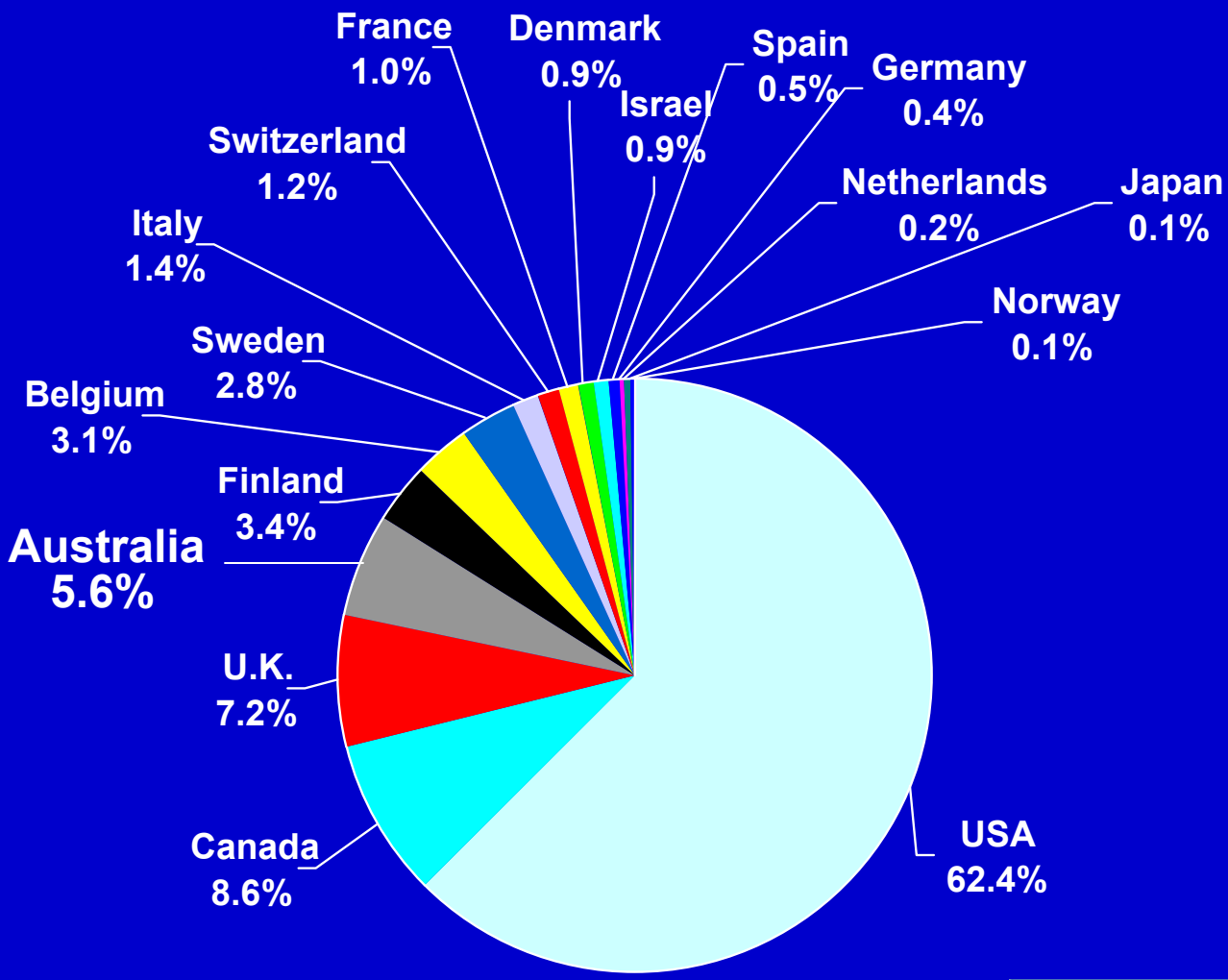
Five-Year Bold and Significant Outcomes on the Path to Cure Type 1 Diabetes

- Generate permanent euglycemia by transplantation of cadaver-based pancreatic islets without chronic immunosuppression
- Generate a replenishable, stable “universal donor” source (xeno, stem cells, etc) of glucose-responsive, insulin-secreting cells that resist immune attack for human transplantation and begin phase I clinical trials
- Restore euglycemia and insulin independence by both activating endogenous beta cell regeneration and inducing immune tolerance to beta cells in animal models of autoimmune diabetes
- Create permanent euglycemia and prevent complications with a closed mechanical loop artificial pancreas
- Develop novel approaches to predict and novel therapeutics to prevent and treat multiple complications
- Accurately predict the risk of type 1 diabetes and develop novel therapeutics to prevent diabetes by maintaining or restoring immune tolerance in new-onset patients with type 1 diabetes

JDRF FY2003:
$81M in research commitments



FY 2003 Funding by Country



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JDRF Clinical Trials:

- Islet transplantation
- New onset T1DM immune intervention

- Autonomic neuropathy
- AGE inhibition
- Antioxidant and hexosamine inhibition

JDRF Clinical Research:

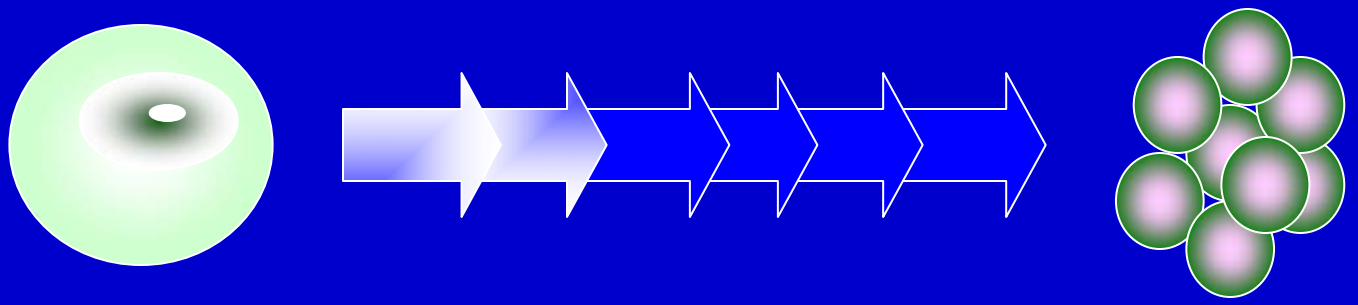
- Genetics of T1DM
- Genetics of Kidney disease
- Immunoassays to monitor autoimmunity
- Hypoglycemia effects on CNS
- Hypoglycemia overnight monitoring in children

JDRF Clinical Trials and Research Issues:

- Endpoints and surrogate markers
- Children involvement
- Enrollment of JDRF constituents and families

Stem Cell Research

- $6M in FY03: $3M ES, $2M adult, $1M animal stem cells ($9M in FY04, ~$20M forward commitments)
- Partnerships: Sweden, UK, France, Finland, Singapore
- Animal-free hES lines being derived
- D. Melton derived 17 lines with JDRF and HHMI funding – in distribution 2004; New lines from partnerships



Stem cell How? β cells

What JDRF desires:

- To derive new stem cell lines useful for therapies
- To characterize existing lines and use them to study beta cell development
- To make stem cell lines freely available to academic researchers
- To promote information exchange and research according to the highest ethical standards

International Stem Cell Forum Organizations:

- Medical Research Council, UK
- National Health and Medical Research Council, Australia
- Canadian Institutes of Health Research
- Academy of Finland
- Inserm, the French Institute of Health and Medical Research
- DFG, German Research Foundation
- Israel Academy of Sciences and Humanities
- Ministry of Education, Culture, Sports, Science and Technology, Japan
- A*STAR Singapore
- Scientific Council for Medicine, Swedish Research Council
- The Netherlands Organization for Scientific Research
- US National Institutes of Health
- JDRF is the only non-government funder

International Stem Cell Forum:

- Brings together leading research agencies from all over the world to discuss working together to support stem cell research
- Objectives of the Forum:
  - To encourage collaborative research across nations, boundaries and disciplines
  - To encourage sharing of resources and data to fully capitalize on the existing available human stem cell lines
  - To identify key research gaps and address these by capitalizing on national strengths
  - To identify funding programs that facilitate transnational collaborations
- Additional information: www.mrc.ac.uk

JDRF: Industry Discovery & Development Partnerships

- JDRF creating target pipeline
- Capitalize on scientific advancements
- Drive novel therapeutic development
- Development requires academic and industry partners