

# ADVANCING RESEARCH TO PATIENTS

research highlights >

DIABETES  
RESEARCH  
INSTITUTE  
FOUNDATION



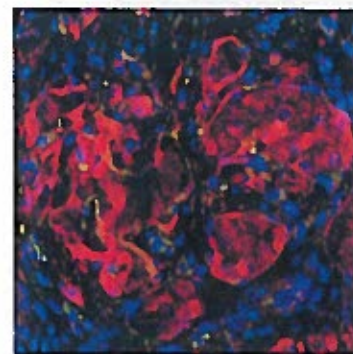
# RESEARCH HIGHLIGHTS: ADVANCING RESEARCH TO PATIENTS

Overcoming the remaining hurdles to discovering a biological cure is the priority at the Diabetes Research Institute and Foundation. DRI scientists are advancing a number of promising initiatives to the clinical phase of testing and gearing up for new patient studies. From testing a tissue-engineered BioHub platform for housing transplanted islets to tackling the complex challenges of the immune system, DRI researchers are intensely focused on translating research to patients in the fastest, safest and most efficient way possible. Below is a summary of five clinical studies that will be underway at the DRI in the coming months:

## BIOHUB TRIAL: ISLET TRANSPLANTATION ONTO THE OMENTUM

As part of its ongoing FDA-approved Phase I/II clinical trial, the DRI's clinical cell transplant team, led by Drs. **Rodolfo Alejandro**, Director of Clinical Islet Transplantation, and **David Baidal**, assistant professor of medicine, produced the first clinical results demonstrating that pancreatic islet cells transplanted within a tissue-engineered platform can successfully engraft and achieve insulin independence in type 1 diabetes. The objective of testing the novel technique is to initially determine if insulin-producing cells can function in this biological scaffold, and then to introduce additional technologies that can protect the cells from immune attack without the need for anti-rejection drugs. The preliminary findings, published in the *New England Journal of Medicine*, are an important step toward developing the DRI BioHub. The results thus far have shown that the omentum is a viable site for islet implantation using the new platform technique. Further evaluation and testing will determine the safety and feasibility of this strategy.

  
The NEW ENGLAND  
JOURNAL of MEDICINE



## DIPIT TRIAL

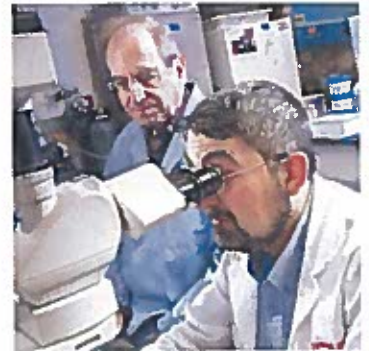


The Diabetes Islet Preservation Immune Treatment (DIPIT) study will combine several different clinically approved agents, given at varying time points, which have demonstrated significant benefits when used alone or in small combinations of two or three of the drugs. This first-ever, multi-combination therapy will test the effects of the agents for halting the immune system attack, preserving the remaining islet function and, possibly, giving the body a chance to recover and regenerate its own insulin-producing cells. Led by Drs. **Camillo Ricordi**, DRI Director, and **Jay Skyler**, Deputy Director for Clinical Research and Academic Programs, the multicenter, Phase I/IIa study will be conducted in a group of patients, ages 18-35, who are within four months of diagnosis with T1D. The data from the study will also provide vital information for developing treatment strategies in people with long-standing type 1 diabetes.



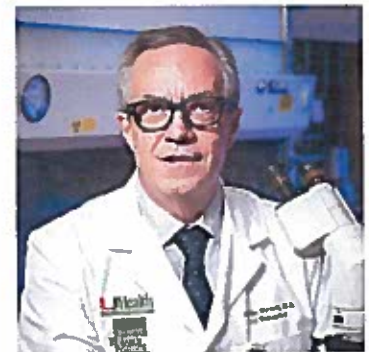
## LOW-DOSE IL-2 IN ESTABLISHED T1D TRIAL

One of the agents used in the DIPIT trial, interleukin-2 (IL-2), is a protein produced by the body that plays a key role in regulating the immune system by boosting the number of desired regulatory T cells (Tregs). Numerous studies conducted by Drs. **Tom Malek**, professor and Interim Chair of Microbiology and Immunology, and **Alberto Pugliese**, Dodson Chair in Diabetes Research and head of the Immunogenetics program, suggest that IL-2 at low doses may correct autoimmunity and help preserve insulin production. Low-dose IL-2 has already been shown to provide clinical benefit to patients affected by other immune-mediated diseases. The FDA-approved Phase I/II trial will test the safety of IL-2 and its effectiveness in improving or preventing further loss of beta cell function. The trial will enroll patients ages 12-21 who are between four months and 12 months post-diagnosis.



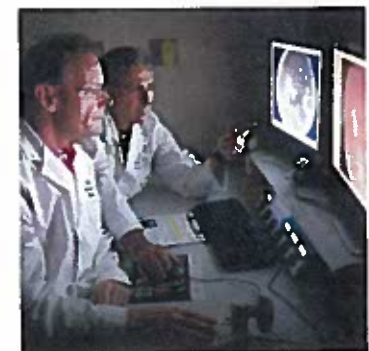
## POSEIDON TRIAL

Several scientific reports have suggested that the use of high-dose omega-3 and vitamin D, both of which have known anti-inflammatory properties, may offer a potential beneficial effect on autoimmune conditions, like type 1 diabetes. DRI researchers hypothesize that the treatment may delay or forestall the disease and will test that theory in a new clinical trial. Pending regulatory approval to proceed, the POSEIDON study (Pilot Study of Omega-3 and Vitamin D High Doses in T1D) will compare the effects of the proposed intervention in children and adults newly diagnosed and in those with longer-standing T1D to evaluate any benefit of early and late interventions. DRI Director Dr. **Camillo Ricordi**, Stacy Joy Goodman Professor of Surgery and Distinguished Professor of Medicine at the University of Miami, and collaborators in Italy recently reported case study results examining the role of omega 3/vitamin D in preserving beta cell function in a young child with type 1 diabetes. The results now warrant further investigation of this potential therapeutic strategy. The DRI will compare children and adults with type 1 diabetes, both newly diagnosed and those more than six months post-diagnosis, who take either vitamin D alone or in combination with omega-3 fatty acids.



## INTRAOCULAR ISLET TRANSPLANT TRIAL

In this clinical trial, researchers will test the safety and efficacy of the eye as a potential site for islet transplantation in a very select group of patients. Drs. **Per-Olof Berggren**, who is the Mary Lou Held Visiting Professor of Surgery at the DRI and was recently elected to the prestigious National Academy of Medicine, and **Midhat Abdulreda**, assistant professor of surgery, are pursuing a novel method to establish immune tolerance by transplanting islets within the anterior chamber of the eye (ACE). Since the eye is recognized as an immune-privileged site in the body, it could offer potential benefits in protecting the transplanted islets from immune attack in the ACE and elsewhere in the body. The researchers have already demonstrated the long-term survival of intraocular (within the ACE) islet transplants without continued anti-rejection drugs in preclinical models. The data suggest two possibilities for establishing long-term islet survival: either through local immune tolerance in the ACE, or as a means to induce peripheral/systemic tolerance whereby islets can be transplanted elsewhere in the body following the initial intraocular transplant. Experiments are currently ongoing to define the immune mechanism(s) contributing to the immunosuppression-free graft survival. In the meantime, the FDA-approved clinical study will test this approach in select patients with type 1 diabetes. This trial will pave the way for future trials aimed at testing immune tolerance induction in pancreatic islet transplants to treat T1D.



# Reaching the Biological Cure >



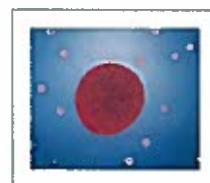
DRI scientists and collaborators have already shown that islet transplantation can restore natural insulin production in people with type 1 diabetes. Some DRI islet transplant recipients have been living without the need for insulin injections for more than a decade, but this life-changing cell replacement therapy remains limited to the most severe cases of type 1 diabetes. DRI scientists are now advancing promising research initiatives with the goal of overcoming these hurdles and offering this freedom to all who need it.

## The DRI BioHub – A Unique Solution for Restoring Natural Insulin Production

One of the Institute's major scientific initiatives is the development of the DRI BioHub, a bioengineered mini-organ that mimics the pancreas. The BioHub builds upon decades of cure-focused research and is a strategy that addresses major research challenges that stand in the way of a biological cure.

### Re-educating the Immune System

Restoring immune system balance to protect insulin-producing cells and prevent the recurrence of the autoimmune process, which mistakenly destroys a person's own cells.



### Co-delivery of "helper" cells

Certain cells in the body that have beneficial properties can be added to help promote long-term islet survival.



### Localized Drug Delivery

Local delivery of low-dose drugs directly into the site can reduce inflammation and protect islets from an immune attack, with the goal of eliminating systemic anti-rejection drugs.





The BioHub strategy can best be grouped into three principal avenues of research, all of which are being pursued simultaneously by the DRI's multidisciplinary team of investigators.

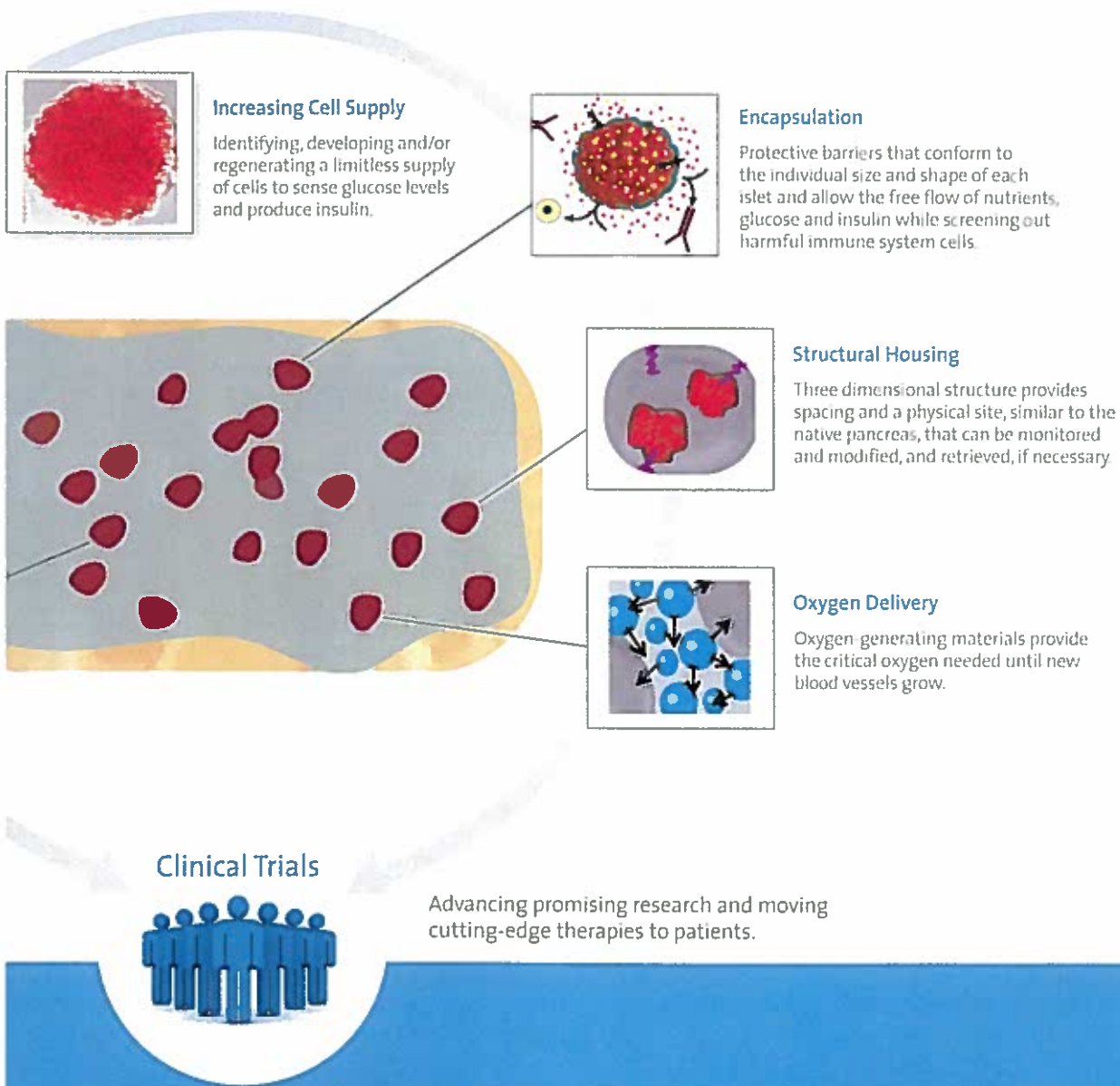
Working together with a global network of like-minded scientists who are committed to a waste-no-time approach, the DRI is integrating the newest cell-based therapies with innovative engineering techniques to restore natural insulin production in those living with diabetes.

These critical research pathways have been collectively referred to as the "Three S's": Site, Sustainability and Supply.

**Site** – Researchers are working to engineer a mini-endocrine pancreas in a site within the body that can house transplanted islets and potentially other "helper" technologies that protect the cells without the need for anti-rejection drugs.

**Sustainability** – To sustain the islets' long-term health and function, researchers are investigating several options. Some methods shield cells using protective barriers, others include adding oxygen or other beneficial agents within the transplant environment. Ultimately, sustainability is about interfering with the autoimmune attack that caused the onset of type 1 diabetes.

**Supply** – Currently, islet cells used for transplantation come from donor pancreases, but there are not enough organs to treat the millions of children and adults living with diabetes. DRI researchers are developing ways to create a reliable and plentiful supply of insulin-producing cells, or even regenerating a patient's own pancreatic cells.



# ABOUT THE DIABETES RESEARCH INSTITUTE FOUNDATION



**MISSION:** To provide the Diabetes Research Institute with the funding necessary to cure diabetes *now*.

The Diabetes Research Institute Foundation (DRIF) is the organization of choice for those who are serious, passionate and committed to curing diabetes. Its mission – to provide the Diabetes Research Institute with the funding necessary to cure diabetes *now* – is a testament to the belief that tomorrow is not soon enough to cure those living with diabetes.



The Diabetes Research Institute has become the world leader it is today through the substantial funding provided by the Foundation. Supported by private philanthropy, the DRIF ensures the jumpstarting of new ideas and the continuation of innovative research projects that remain cure-focused and will ultimately benefit those with diabetes.

The DRIF's history of commitment dates back to 1971 when it was founded by a small group of parents of children with diabetes who were dedicated to finding a cure. Driven by a shared mission, they banded together to support a promising research program at the University of Miami solely aimed at curing those living with diabetes. In an unprecedented partnership that spans more than three decades and continues today, North America's Building Trades Unions joined with the Foundation's leadership to help fulfill its mission to cure diabetes. The DRIF's largest contributor, the Building Trades committed to funding – and building – the Diabetes Research Institute facility. The unions have raised more than \$50 million for the DRI and today, under the banner of Blueprint for Cure, thousands of union members undertake fundraising projects nationwide to provide ongoing support.

The DRI Foundation is recognized as one of the world's most respected diabetes organizations. Garnering the attention of influential people who are personally affected by diabetes, the Foundation has grown into an international coalition of business leaders, celebrities, scientists, clinicians, families and other concerned individuals who have elevated the importance of cure-focused research and provided meaningful support for the DRI's multidisciplinary research program.

This funding is provided through individual and corporate donations, special events, sponsorships, cause marketing relationships and planned giving, which allows donors to provide a gift in the form of a will, trust or other deferred giving vehicle.

In an effort to increase awareness about the latest advances toward a cure, the Foundation conducts a wide variety of activities both online and offline, hosts research updates and workshops for people with diabetes and their families, and produces numerous printed publications and e-communications to make this information accessible to people nationally and internationally.

A 501(c)(3) not-for-profit corporation, the DRI Foundation has thousands of supporters in the United States and worldwide, and, in addition to its headquarters in Florida, operates regional development offices in New York, Long Island and Washington, D.C.

The Diabetes Research Institute Foundation was created for one reason – to cure diabetes – which is and will continue to be its singular focus until that goal is reached. For the millions of individuals and families affected by diabetes, the Diabetes Research Institute Foundation is the best hope for a cure.



**National Office  
Florida Region**

200 South Park Road  
Suite 100  
Hollywood, FL 33021  
Telephone 954.964.4040  
Toll-free 1.800.321.3437  
Fax 954.964.7036  
info@drif.org

**Northeast Region**

Jericho Office  
400 Jericho Turnpike  
Suite 326  
Jericho, NY 11753  
Telephone 516.822.1700  
Fax 516.822.3570  
neregion@drif.org

Manhattan Office  
259 West 30th Street  
Suite 402  
New York, NY 10001  
Telephone 212.888.2217  
Fax 212.888.2219  
neregion@drif.org



DiabetesResearch.org

Designed by francis@fr