

# Pediatric Diabetes Research Center

UC San Diego  
HEALTH SCIENCES

NEWSLETTER

FALL 2014

## A MESSAGE FROM THE PDRC DIRECTOR

Thank you for being a part of our community of supporters!

Located in the hub of San Diego's biotech hotbed, UC San Diego's Pediatric Diabetes Research Center (PDRC) boasts a unique blend of top-ranked physicians, research scientists, world-class laboratories, and innovative therapies all with one goal - ending type 1 diabetes (T1D).

It is our mission to improve the quality of life for those with diabetes through groundbreaking research in the prevention, treatment and cure of type 1 diabetes and by providing optimal, compassionate clinical care and education.

We would like to thank Lisa and Steve Altman for once again hosting the annual Rock the Cure event at their home in support of the PDRC. This year's event featured rock legends Foreigner and was attended by more than 500 supporters. We are delighted to report that the event raised nearly \$850,000 to support the PDRC. We remain indebted to all those who attended and have so generously supported us.

We hope you find the following information about our researchers' groundbreaking and innovative work as interesting, and exciting, as we do. Thanks to the support of our partners and friends in the community, we continue moving toward a future where T1D is a disease of the past!

*Maïke Sander, MD*



## INTRODUCING WENXIAN FU, PHD

We are delighted to announce that Dr. Wenxian Fu joined the PDRC last November from Harvard University in Boston. He studies the immune regulatory mechanisms that lead to type 1 diabetes (T1D) with the goal of discovering new preventive and therapeutic strategies.

In people with T1D, the patient's own immune system destroys the beta cells in the pancreas which produce insulin, a vital hormone necessary for keeping blood sugar levels normal. Dr. Fu's research is focused on the early identification of abnormalities in the pancreas before patients develop diabetes. He hopes to translate those findings into novel preventive strategies that will suppress the autoimmune attack on beta-cells before the onset of clinical symptoms.

Dr. Fu is also exploring new imaging methods, such as Magnetic Resonance Imaging (MRI), for predicting the risk of developing T1D before patients are diagnosed with the disease. He has already used these imaging techniques in animal models to detect early signs of the autoimmune attack. Translating these imaging methods to clinical care and combining them with other screening methods for T1D risk could lead to much greater accuracy for predicting T1D and provide opportunities for early therapeutic interventions.

Visit the PDRC website or click [here](#) to read more about Dr. Fu's research.



## IDENTIFYING STRATEGIES TO PREVENT BETA CELL DESTRUCTION

In type 1 diabetes, the immune system directs the insulin-secreting beta cells to self-destruct. PDRC researcher Dr. Ulupi Jhala has developed a novel culture model in which this process of autoimmune beta cell destruction is recreated "in the dish" using human islets and immune cells.

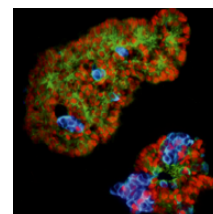
Using this model, Dr. Jhala has studied the mechanisms that result in beta cell suicide. Many of the factors involved in the cross talk between immune cells and beta cells are highly responsive to drugs. Dr. Jhala has identified a class of these factors and is collaborating with a local biotech company to examine whether pharmacologic inhibition of these factors can improve beta cell survival and function.

Additionally, Dr. Jhala is using the same culture model to identify stable byproducts released during the destruction of beta cells. By studying these byproducts, Dr. Jhala hopes to identify a unique signature of beta cell destruction that could be detected long before the development of diabetes, when therapeutic intervention may still be possible. The long-term goal is to develop better diagnostic tests to identify the very early stages of beta cell decline when therapeutic rescue of beta cells may still be possible.



## ADVANCING THE DEVELOPMENT OF STEM CELL BASED THERAPIES FOR DIABETES

PDRC Director Dr. Maïke Sander was recently awarded two grants from the California Institute of Regenerative Medicine (CIRM) to advance the development of stem cell-based therapies for diabetes. Individuals with type 1 diabetes could benefit tremendously from the availability of transplantable replacement beta cells and one approach is to produce beta cells from stem cells. However, it is not possible to produce functional beta cells from stem cells in the culture dish at this time.



By studying the molecular cues that regulate how beta cells develop and mature, Dr. Sander will examine why beta cells are not appropriately produced under current culture conditions. Results from these studies will be critical in improving current approaches for generating functional beta cells in the lab for cell replacement therapies.

Visit our website or click [here](#) to read more about the research underway at the PDRC.

## BRINGING THE LATEST DISCOVERIES FROM THE LAB TO THE CLINIC

There are multiple ongoing clinical studies for individuals with type 1 diabetes (T1D) at the PDRC. Participants in clinical trials help researchers learn more about the effectiveness of new therapies and benefit from ongoing contact with the Rady Diabetes Care Team.

### Featured studies:

#### ***TrialNet Pathway to Prevention***

Purpose: Identify relatives of type 1 diabetics that have positive antibodies. Participants with positive antibodies will be offered preventative studies and/or be closely monitored for the onset of diabetes.

Recruiting: 1) Individuals 1 - 45 years old who have an immediate family member with T1D (such as a child, parent or sibling); 2) Individuals 1 - 20 years old who have an extended family member with type 1 diabetes (such as a cousin, niece, nephew, aunt, uncle, grandparent or half-sibling).

*(Continued on page 3)*



(Continued from page 2)

### **Grifols Study**

Purpose: Evaluate the safety and efficacy of four dosing regimens of human plasma-derived alpha1-PI. Subjects would be treated with intravenous study drug or placebo (no real drug) weekly for 13 or 26 weeks.

Recruiting: Individuals 6 to 35 years old who are within three months of being newly diagnosed with T1D.

### **Metformin T1D Study**

Purpose: Evaluate the efficacy and safety of the use of metformin in addition to standard insulin therapy in overweight and obese children and adolescents, age 12 - <20 years, with T1D (*recruitment is closed*).

### **T1D Exchange**

Purpose: Improve our knowledge and understanding of the optimal approaches to the management of T1D in children and adults through sharing of best practices by collecting data in a common data repository (*recruitment is closed*).

Visit our website or click [here](#) to read more about clinical trials at the PDRC.

## **DIABETES CLINICAL CARE UPDATE**

### ***Diabetes Program Ranked Among the Nation's Best***

The PDRC is very excited to announce that this year the UC San Diego/Rady Children's Pediatric Endocrinology Diabetes division was rated number 12 in the country by U.S. News & Report's annual survey of children's hospitals. This ranking has steadily moved up from number 28 in 2009 when Rady Children's Hospital first began submitting data to the survey. Last year the program was rated number 16. Most of the questions in the survey are focused on the care of patients with diabetes. In the category of diabetes patient management, the division received the top score. Only two other programs in the top 12 positions have achieved this distinction.



Every year the diabetes clinic continues to increase in volume and in services offered. Over the past year more than 1,300 children with diabetes were cared for by the diabetes team which includes nurse educators, dietitians, social workers and physicians. The insulin pump and continuous glucose monitoring sensor programs continue to expand as more patients begin utilizing this technology. Earlier this year, the diabetes clinic relocated to a new, larger site at the Rady Children's Hospital campus that includes 16 examination rooms, two education rooms, one clinical research room and office space.

### ***New Retinal Camera Makes Yearly Eye Exams Easier***

Soon patients will be able to have their yearly retinal (back of the eye) examination performed in the clinic during routine care visits. The diabetes clinic has obtained a retinal camera for monitoring potential early changes in the eyes of patients with diabetes. The system is very simple for taking pictures of the retina, takes less than a minute and it does not require the eyes to be dilated. The photographs will be reviewed by a pediatric ophthalmologist.

The PDRC would not be possible without the support of our extraordinary donors. Gifts made to the PDRC help researchers get closer to our goal: a cure for diabetes. If you would like to make a donation to support the PDRC, visit our website or click [here](#) to donate online or call Brian Zumbano at 858-246-1557 for more information.

**Pediatric Diabetes Research Center (PDRC)**  
Visit our website: [pdrc.ucsd.edu](http://pdrc.ucsd.edu)

University of California San Diego / 9500 Gilman Drive, #0983 / La Jolla, CA 92093 Email: [pdrc@ucsd.edu](mailto:pdrc@ucsd.edu)