



# MILLION DOLLAR BIKE RIDE GRANT WINNERS

## 2014

### RESEARCH TOPIC:

Pilot Study of the Efficacy and Safety of Sirolimus in the Treatment of Congenital Hyperinsulinism

Inactivating mutations of the KATP channels cause the most common and most severe form of HI (KATPHI). Children with KATPHI are unresponsive to medical therapy and require pancreatectomy to control hypoglycemia. After pancreatectomy, 50% of children with diffuse HI continue to have hypoglycemia and by puberty, 91% of them have developed diabetes. Thus, there is a significant unmet need for more effective medical treatments for KATPHI.

Sirolimus has shown potential promise as a treatment for KATPHI. A carefully controlled study of the efficacy and safety of sirolimus for HI is needed before being adapted as a standard therapy.

This is a pilot study to determine the efficacy, safety and tolerability of sirolimus in infants with KATPHI. The primary endpoint of the study is the number of subjects off intravenous dextrose support at the end of the study period. This pilot study is a critical step potentially leading to a large multicenter study of the efficacy of sirolimus in infants with KATPHI.

*"I am riding for research because new treatments are desperately needed to improve the outcomes of children with hyperinsulinism," says Dr. De León.*

AWARD RECIPIENT:

Dr. Diva De León,  
MD, MSCE

HOME INSTITUTION:

Children's Hospital  
of Philadelphia

AWARD AMOUNT:

\$60,000



**RIDING FOR RESEARCH!**



**Penn Medicine**  
Orphan Disease Center