



MILLION DOLLAR BIKE RIDE GRANT WINNERS

2016

RESEARCH TOPIC:

Drug development for Treatment
of Glutamate Dehydrogenase
Hyperinsulinism

Glutamate

dehydrogenase hyperinsulinism (GDH-HI) is the second most common form of HI. Like all people with HI, if untreated, those with GDH secrete too much insulin and, consequently, suffer from hypoglycemia. They are especially at risk for low blood sugar after a protein rich meal. They have high ammonia levels in their blood, epilepsy and other health issues. Currently they are treated with diazoxide for HI, but this only treats the symptoms and not the cause of the disease.

The goal of this study is to identify a safe medicine to treat all the health issues resulting from activating mutations in GDH in people with GDH-HI. In early investigations, we identified that a component of green tea extract, EGCG, is a potent inhibitor of GDH and we have also identified several other lead GDH inhibitors. A GDH inhibitor would be an ideal treatment, because it would control the excess insulin secretion, and could improve liver, kidney and brain function by controlling hyperactive GDH enzyme.

"Hyperinsulinism research will help us to understand the mechanisms of the disease and is the only way to find better treatment," Dr. Li says, on his passion for HI research!

AWARD RECIPIENT:

Changhong Li
MD, PhD

HOME INSTITUTION:

Children's Hospital
of Philadelphia

AWARD AMOUNT:

\$82,000



RIDING FOR RESEARCH!



Penn Medicine
Orphan Disease Center