

# Finding Solutions in Combating Mitochondrial Diseases



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## CURRENT RESEARCH

### Clinical trials are proving positive with Dichloroacetate (DCA)

Mitochondria are the intracellular "powerhouses" of our cells. They are responsible for generating the energy needed by every tissue and organ in our bodies to perform their normal functions. Energy is essential to life and, when energy production is compromised, disease results. PDC is a key enzyme for maintaining the body's energy supply. The scientific team lead by Dr. Peter Stacpoole at the University of Florida in Gainesville, Florida, has connected a number of disease states to their potential treatment with the drug dichloroacetate (DCA). DCA stimulates PDC, increasing its ability to promote cellular energy production. DCA has shown promise in treating several life-threatening diseases, including cancer, pulmonary arterial hypertension and congenital PDC deficiency (PDCD) in children.

Solutions are needed to deliver the fruits of science to patients for whom they are intended. With DCA, Dr. Stacpoole's team has developed a uniquely acting compound that is a prototype of new class of drugs to increase the efficiency of normal metabolic processes essential for cell survival. Indeed, the story of DCA is a striking example in which the basic scientific questions have been answered and animal studies and even early stage clinical trials have been conducted. Yet, DCA is too simple a molecule to be patented. This problem has prevented traditional pharmaceutical support for conducting human trials with DCA in diseases in which currently approved therapy is either inadequate or nonexistent.

- Dr. Stacpoole and his team at the University of Florida are among the few researchers in academia whose efforts in drug development have advanced to the stage of human trials. They have worked with the FDA...

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## AFFILIATION

 University of Florida

## EDUCATION

- Ph.D. in Pharmacology 1972 , University of California, San Francisco
- Post-Doctoral Training in Metabolic Research 1974 , University of California, San Francisco
- M.D. in Medicine 1976 , Vanderbilt University

## AWARDS

- The American Society for Clinical Investigation

## RESEARCH AREAS

Life Science, Oncology / Cancer, Genomics / Congenital

## FUNDING REQUEST

Your contributions will help Dr. Stacpoole and his team to save and extend the lives of infants, children, and adults with rare and common diseases worldwide. Give his team the power to treat congenital PDC deficiency, further the use of DCA to eradicate tumor cells, and implement the use of DCA against PAH which attacks patient's lung and cardiac function. By funding Dr. Stacpoole you can help provide the support needed to continue clinical trials for cancer patients, pulmonary hypertension patients, and PDC deficient patients.