



## TECHNOLOGY

# Use of Trehalose for Prevention of Neural Tube Defects

## OVERVIEW

The neural tube is a specialized part of the embryo that forms the skull, brain and spinal cord in adults and requires an effective balance between cell death and survival. Disruption of this process causes neural tube defects (NTDs) such as spina bifida, anencephaly, and encephalocele. UMB researchers have discovered Trehalose treatment reverses autophagy impairment and prevents NTD in conditions, such as, diabetic pregnancies. Unmanaged diabetes before and during pregnancy can disrupt the process of degradation of unnecessary or dysfunctional cellular components (autophagy) and induce neural tube malformation in the fetus. Maternal hyperglycemia, mood stabilizing drugs (lithium, valproate, zoloft etc), epilepsy treatment drugs, folate deficiency, chromosomal abnormalities and gene mutations are some of the known causes of NTDs. Trehalose-induced autophagy restores homeostatic balance by correcting mitochondrial defects, dysfunctional proteins, endoplasmic reticulum (ER) stress, apoptosis, and delayed neurogenesis in the neural tube exposed to maternal hyperglycemia. These findings provide evidence for the potential efficacy of trehalose, a non-toxic natural sugar, as an intervention against maternal hyperglycemia-induced NTD. Accordingly, the invention provides methods for preventing hyperglycemia-induced NTD, by administering trehalose to pregnant women with hyperglycemia due to diabetes or other causes.

## APPLICATIONS

Neural tube defects are one of the most common birth defects, occurring in 1/1000 births in the USA. The lifetime costs for care of a child born with a NTD are high. The estimated yearly medical and surgical costs for children born with spina bifida have gone up from US \$81 to \$200 million in the last 20 years. National per capita medical costs for spina bifida alone can exceed \$1 million, with additional direct and indirect costs accruing over time. The invention has the potential to address two overlapping market problems and needs: 1. Diabetes-induced birth defects in infants (neural malformations, heart defects and still birth). 2. Neural Tube Defects (NTDs), which lead to infant mortality or severe lifelong disability. Thus, there is an unmet need for effective and safe strategies to interfere with the mechanism and reduce the occurrence of such birth defects.

## ADVANTAGES

Trehalose: -Restores maternal diabetes-impaired autophagy and prevents NTD formation. -Corrects maternal diabetes-altered gene expression that regulates autophagy. -Suppresses maternal diabetes-induced ER chaperone gene expression. -Abolishes maternal diabetes-induced caspase 3, 8 cleavage. -Blocks maternal diabetes-induced neuroepithelial cell apoptosis, Bcl2 associated death promoter (Bad), and BH3 interacting domain death agonist (BID) activation.

## R&D REQUIRED

Additional safety studies and formulation required.

## LICENSING POTENTIAL

UM seeks to develop and commercialize by an exclusive or non-exclusive license agreement and/or sponsored research with a company active in the area.

## **CONTACT INFO**

Office of Technology Transfer  
620 W Lexington St., 4th Floor  
Baltimore, MD 21201  
Email: [ott@umaryland.edu](mailto:ott@umaryland.edu)  
Phone: (410) 706-2380

## **Additional Information**

### **INSTITUTION**

University of Maryland, Baltimore

### **PATENT STATUS**

US Patent Application filed, pending

### **CATEGORIES**

- Therapeutics
- Small molecules

### **INVESTIGATOR(S)**

Peixin Yang  
E. Albert Reece

### **EXTERNAL RESOURCES**

- [Trehalose prevents neural tube defects by correcting maternal diabetes-suppressed autophagy and neurogenesis.](#)

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