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NEWS RELEASES

Media Advisory

Wednesday, September 9, 2020

Miglustat improves swallowing in children and adolescents with Niemann-Pick type C1 disease

NIH observational study suggests that the drug may decrease risk of pneumonia and death in this population.

What

The drug miglustat appears to stabilize the swallowing problems that occur in children and adolescents with Niemann-Pick type C1 (NPC1), a rare and ultimately fatal neurological disease, according to a study by researchers at the National Institutes of Health. The authors conclude that the drug could slow the deterioration of swallowing function in NPC1 cases and decrease the risk of pneumonia resulting from aspiration, or inhaling food or drink. Aspiration pneumonia accounts for roughly 2 out of 3 deaths in people with NPC1.

The study was conducted by Forbes D. Porter, M.D., Ph.D., of NIH's *Eunice Kennedy Shriver* National Institute of Child Health and Human Development and colleague in the NIH Clinical Center. It appears in the *JAMA Neurology*.

NPC1 is a rare genetic disorder that causes a progressive decline in neurological and cognitive functions. Although miglustat is not approved by the Food and Drug Administration to treat NPC1, the drug is thought to stabilize the neurological deterioration seen in the disease and is frequently prescribed to treat it. Previous studies have suggested that by slowing this neurological deterioration, miglustat can stabilize swallowing ability. However, these studies have not documented any specific swallowing improvements for patients.

In the current study, researchers used video fluoroscopy — an X-ray scan of the throat — to evaluate swallowing function in 120 NPC1 patients. They ranked each of the scans based on the need for feeding tubes and other interventions to assist swallowing and on the likelihood and extent of food and drink entering the airway. On average, patients were evaluated once a year for three years. Of these, 36 had been prescribed miglustat and 24 had not. The researchers found that, compared to those not taking miglustat, those who took the drug had a 91% lower risk for deterioration in swallowing and a 72% lower risk for getting food or drink in the airway.

WHO

Forbes D. Porter, M.D., Ph.D., senior investigator at the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development, is available for comment.

Article

Solomon, BL et al. Miglustat stabilizes swallowing outcomes in a Niemann-Pick disease, type C1 observational study. *JAMA Neurology*.2020.

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