Miglustat (Zavesca®)

This sheet is about exposure to miglustat in pregnancy and while breastfeeding. This information is based on published research studies. It should not take the place of medical care and advice from your healthcare provider.

What is miglustat?

Miglustat is a medication that has been used for treatment of mild to moderate Gaucher disease type 1. It has also been used to treat Niemann-Pick disease type C. Miglustat is sold under the brand names Zavesca®, Opfolda®, and Yargesa®.

People with Gaucher disease have low levels of an enzyme called glucocerebrosidase. This enzyme helps break down fatty substances in the body. When this enzyme is missing or not working, fatty substances build up and can cause organ damage. Miglustat works in the body to limit the amount of fatty substances being made. For more information, see the MotherToBaby fact sheet on Gaucher disease at https://mothertobaby.org/fact-sheets/gaucher-disease-pregnancy/.

Sometimes when women find out they are pregnant, they think about changing how they take their medication, or stopping their medication altogether. However, it is important to talk with your healthcare providers before making any changes to how you take your medication. Your healthcare providers can talk with you about the benefits of treating your condition and the risks of untreated illness during pregnancy.

I take miglustat. Can it make it harder for me to get pregnant?

Studies have not been done in humans to see if miglustat could make it harder to get pregnant.

Does taking miglustat increase the chance of miscarriage?

Miscarriage is common and can occur in any pregnancy for many different reasons. Studies have not been done in humans to see if miglustat could increase the chance of miscarriage. Studies in animals found a greater chance of pregnancy loss at doses around twice as much as would be used in human therapy.

Does taking miglustat increase the chance of birth defects?

Birth defects can happen in any pregnancy for different reasons. Out of all babies born each year, about 3 out of 100 (3%) will have a birth defect. We look at research studies to try to understand if an exposure, like miglustat, might increase the chance of birth defects in a pregnancy.

Studies have not been done in humans to see if miglustat can increase the chance of birth defects. Animal studies done by the manufacturer did not find an increased chance of births defects.

Does taking miglustat in pregnancy increase the chance of other pregnancy-related problems?

Studies have not been done in humans to see if miglustat can increase the chance of pregnancy-related problems such as preterm delivery (birth before week 37) or low birth weight (weighing less than 5 pounds, 8 ounces [2500 grams] at birth). Animal studies done by the manufacturer reported a higher chance of low birth weight.

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oes taking miglustat in pregnancy affect future behavior or learning for the child?

Studies have not been done in humans to see if miglustat can affect future behavior or learning for the child.

Breastfeeding while taking miglustat:

There are no studies looking at miglustat use while breastfeeding. The product label for miglustat states that because there is no data available, use during breastfeeding is not recommended. But the benefit of using miglustat may outweigh possible risks. Your healthcare providers can talk with you about using miglustat and what treatment is best for you. Be sure to talk to your healthcare provider about all your breastfeeding questions.

If a man takes miglustat, could it affect fertility or increase the chance of birth defects?

Fertility (ability to get a woman pregnant) was not affected in 7 men treated with miglustat. Animal studies in rats found that miglustat exposure lowered sperm production, which lowered fertility. However, this was not found in all animal studies. In general, exposures that fathers or sperm donors have are unlikely to increase risks to a pregnancy. For more information, please see the MotherToBaby fact sheet Paternal Exposures at https://mothertobaby.org/fact-sheets/paternal-exposures-pregnancy/.

Please click **here** for references.

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