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Researchers find mechanism that could prevent or treat deadly peroxisome diseases

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University of Alberta medical researchers have made a major breakthrough in understanding a group of deadly disorders that includes the disease made famous in the movie Lorenzo's Oil.

Because this group of diseases is inherited, the discovery could help in screening carriers and lead to prevention or an effective treatment.

Richard Rachubinski, in the Faculty of Medicine & Dentistry, is an expert on structures in cells called peroxisomes which are involved in breaking down fatty acids. They are vital for humans. Babies born with a peroxizome disorder do not typically survive longer than a year because of impaired metabolism.

In his latest study, Rachubinski found another clue in the search to understand the peroxisome and the disorders caused by its malfunction. He and his team discovered that a protein family thought to be only involved in the early stages of making peroxisomes is actually crucial in ensuring peroxisomes transfer into other cells after they divide. All cells of the body must have peroxisomes to survive. These proteins are found in every living being, so they provide a universal mechanism not only for how peroxisomes are made but also for how peroxisomes are maintained in cells to keep them alive as they divide.

One peroxisome disorder is adrenoleukodystrophy, or ALD, the disease a boy named Lorenzo Odone suffered from. His parents' fight for a cure was made into the movie.

Rachubinski's findings have been published in the Journal of Cell Biology.

Provided by University of Alberta

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