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**Press release**  
**For immediate publication**

**Rare disease can cause liver inflammation**  
**Med Uni Graz researches promising treatment with lanifibranor**

Graz, 06 August 2024: Millions of people worldwide suffer from liver inflammation (hepatitis), an acute or chronic disease with a variety of causes. Liver inflammation frequently occurs in connection with metabolic disorders, for example fatty liver disease. Lysosomal acid lipase (LAL) deficiency is a rare metabolic disorder that also causes liver inflammation. A paper recently published by Medical University of Graz scientists presents a promising method for treating liver inflammation due to LAL deficiency. The drug lanifibranor has the potential to reverse metabolic and inflammatory processes in the liver involved in the development of liver inflammation.

**Lanifibranor: New drug helps the “recycling centers” of cells**

Lysosomal acid lipase (LAL) deficiency is a very rare genetic metabolic disorder caused by a deficiency in the lysosomal acid lipase (LAL) enzyme. This enzyme is required to break down certain fats, such as cholesteryl esters and triglycerides, within the lysosomes, the “recycling centers” of the cells. “If these fats in the cells are not broken down, the result is inflammation, which can greatly impact the liver,” explains Dagmar Kratky of the Gottfried Schatz Research Center at the Medical University of Graz. The LAL deficiency develops due to a genetic mutation that leads to reduced or missing enzyme activity.

These recent Med Uni Graz research findings suggest that the drug lanifibranor has positive effects on liver inflammation and metabolic disorders due to LAL deficiency. “People deficient in LAL could benefit from new methods for treating the disorder since only (extremely expensive) enzyme replacement therapy has been partially successful,” says Dagmar Kratky. With colleagues in Graz and Copenhagen, the lipid metabolism expert has studied how treatment of LAL deficiency with lanifibranor works.

## Reduces liver damage and improves blood lipid levels associated with LAL deficiency

In one study, mice with LAL deficiency were treated with lanifibranor once a day for 21 days. The findings show that the treatment slightly changed the weight of the organs while the liver lipid values remained the same, yet it reduced signs of liver damage and proteins associated with inflammation in the liver. In addition, the share of proteins involved in energy production in cells rose and blood lipid levels improved.

The positive effects of lanifibranor on liver inflammation and the improved blood lipid levels indicate that a combination of lanifibranor and enzyme replacement therapy for LAL deficiency may represent a promising new treatment. These findings underline the necessity for further studies and clinical tests in order to evaluate the effectiveness of lanifibranor as a potential treatment option for LAL deficiency in humans. "These new findings offer hope for an additional and improved treatment option for patients with LAL deficiency and may represent an important step in medical research," concludes Dagmar Kratky.

### To the publication

*Lanifibranor reduces inflammation and improves dyslipidemia in lysosomal acid lipase-deficient mice*

<https://www.sciencedirect.com/science/article/pii/S2772572324000736?via%3Dihub>

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### Profile of Dagmar Kratky

Dagmar Kratky is a biochemist and molecular biologist specializing in fat and energy metabolism. Her research group investigates the role of fat-splitting enzymes, so-called lipid hydrolases, in cells and organs. The aim of her research is to counteract metabolic disorders by identifying the molecular mechanisms that cause metabolic abnormalities.