

Tim Barrett update for Winter TreatWolfram newsletter

Dear friends and colleagues,

I hope everyone is keeping OK and are keeping warm enough in this cold weather. Our research teams have kept busy and I would like to use this letter to keep you updated on our progress.

The TREATWOLFRAM trial is now in its final year. It has been a long journey to get here, navigating medicine manufacture problems, BREXIT, Covid, and supply issues. Thank you to all the participants who have supported the trial with their involvement. Our trials unit team, Amy Lamb, Lewis James Victoria Homer and Darren Barton, are working hard to make sure all the information that participants have kindly supplied, will be ready to be analysed when the trial ends in October 2024. There has been an update from the UK Medicines Regulator (MHRA) to remind patients and the public about the safety precautions we all need to follow for people taking sodium valproate. Anyone female under 55 years who may be taking sodium valproate, needs to have a safety check with their doctor each year. It is important that sodium valproate should not be taken by women who may be pregnant, as there is a risk of significant harm to the baby. There is helpful information on this UK Government website: <https://www.gov.uk/guidance/valproate-use-by-women-and-girls> .

Our laboratory team, led by Dr Sovan Sarkar, with Dr Malgosia Zatyka, and their colleagues, have been finding new biological pathways in cells that are important in developing Wolfram syndrome. Some of these pathways relate to the ways that cells clear unwanted by-products. Sovan's team have studied these pathways to look for new treatments to correct the defects. They have some candidate medicines, and have applied to research funders to test if these medicines work in cell models. Sovan's team presented at a major conference hosted in Birmingham at the end of the Summer, and one of his team members won the prize for best presentation.

Finally, our team has been approached by researchers in Europe and North America, to support their work on Wolfram syndrome. The team in North America are looking at changes in DNA that silence or activate genes and how these affect symptoms in Wolfram syndrome. The team in Europe are hoping to set up a new clinical trial of a treatment for Wolfram, and we are sharing our expertise, data and samples to help push this research forward

All our study team owes a big thank you to Wolfram syndrome UK for their generous support, and for bringing the Wolfram community of families, researchers and health care professionals together.

Sincerely