

LI FRAUMENI SYNDROME (LFS)



WHAT IS LFS?

Li Fraumeni Syndrome (LFS) is caused by a mutation in the Tp53 gene, which codes for a protein (called p53) which suppresses tumour growth. p53 regulates the cell cycle and prevents cells from dividing uncontrollably to form a tumour.

People with LFS have a 50% chance of developing cancer before the age of 40, rising to 90% by the age of 60. Associated cancers include bone and soft tissue sarcomas, acute leukaemia, as well as breast, brain, and adrenal cancers.

People with LFS are treated for each cancer they develop in the same way as other patients. There are currently no LFS-specific treatments.

METFORMIN CLINICAL TRIALS

Metformin, a drug used to control blood sugar levels in people with type II diabetes, has been correlated with lower cancer risk in numerous clinical trials.

Mouse studies suggest metformin changes the metabolism of cells with the TP53 gene mutation, making them behave more like normal cells. This reduces the likelihood of cancer.

The Metformin for Li Fraumeni (MILI) clinical trial is now testing the effect in humans. The 5-year Phase II study started in 2024 and will track two groups of patients, those on metformin and those not, to determine if the drug can prevent cancer in Li Fraumeni patients.

SECURING FUNDING

SCIENTIFIC RATIONALE AND DATA

Researchers used mouse studies to show that metformin was effective. Additionally, as a repurposed drug, metformin's safety and toxicity are well understood and documented. A robust biological rationale and high-quality safety data support successful funding applications for clinical trials.

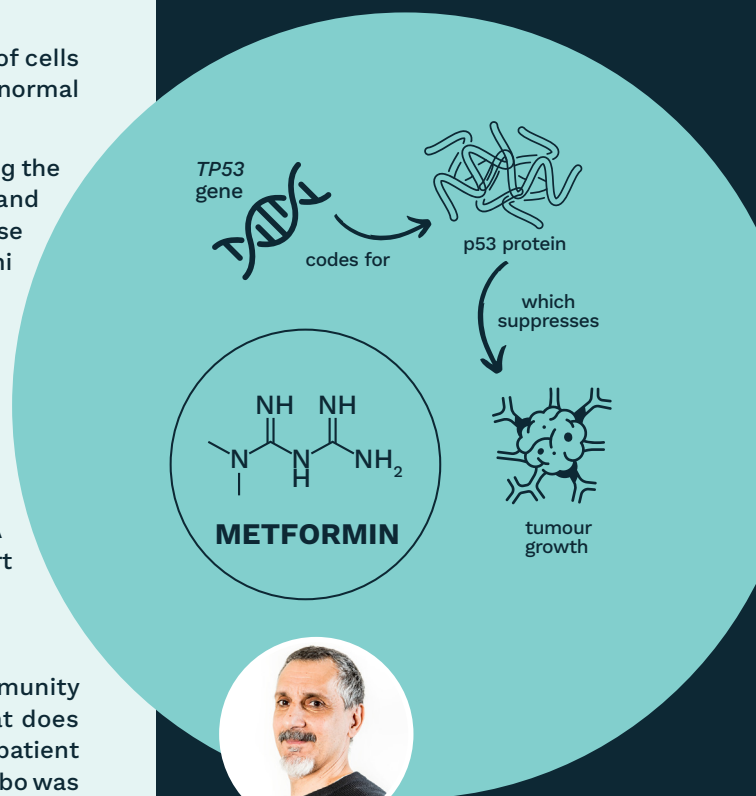
WORKING WITH PATIENTS

In the UK branch of the trial, known as MILI, the patient community did not want to take a placebo, i.e. a control substance that does not contain the medication. A high level of engagement with patient advocates was instrumental in convincing funders that a placebo was not essential in this trial. It is crucial to engage with patients to secure the most appropriate clinical protocols while obtaining high-quality scientific data.

AT A GLANCE

- LFS increases a person's risk of developing some types of cancer.
- Metformin is a drug used to help diabetic people.
- It is being trialled to see if it can have an impact in reducing cancer rates in people with LFS.
- Having robust preclinical data and good relationships with funders can help you secure funding for repurposing trials.

HOW IT WORKS



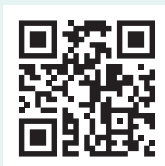
SPECIAL THANKS TO PAN PANTZIARKA

Pan is the Programme Director of Drug Repurposing for the Anticancer Fund, as well as chairperson of the George Pantziarka TP53 Trust. He's a key driver in the metformin clinical trial.



REMEDIA4ALL is funded by the European Union's Horizon Europe research and innovation programme under grant agreement No 101057442.

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