

CASE STUDY: She said yes!

PSR's orphan team helped a Wilson's Disease patient to successfully manage her trial participation on her honeymoon

A phase II/III pivotal study in Wilson's Disease

- 40 adult patients, newly-diagnosed with Wilson's Disease
- 9 study sites, 5 countries (US, UK, DE, PL, AU)
- Full services provided by PSR

Case Study Challenge & Mitigations

Case: A 32-year old woman at one of the UK-sites who was recently diagnosed with Wilson's Disease and was willing to participate in a trial/study for people with Wilson's disease.

Challenge: The patient's wedding and 3-week honeymoon in Cyprus was arranged for 1 month after her study-enrolment. Since this was considered a critical period for the safety tests of the trial/study (particularly the risk of elevated LFTs), it was crucial to arrange weekly blood-draws for safety-labs during her planned wedding and honeymoon.

Mitigation: PSR contacted a private clinic in Cyprus and was able to arrange that the patient could get her study weekly blood-draws while on her honeymoon at her resort. Samples were analyzed by the local clinic in Cyprus (routine hematology/biochemistry) and results were e-mailed to the investigator in the UK, so he could assess whether the results indicated any change to the study/trial IMP-dose.

Luckily and thanks to PSR's proactivity, the patient's LFTs remained stable while getting married, enjoying her honeymoon, and continuing the clinical trial.

PSR orphan drug service engagement take in

- PSR treats any rare-disease patient like gold-dust and will do anything in our power to enroll a rare-disease patient into a trial or to try to prevent a patient from dropping out or stopping their trial participation
- PSR likes to think out-of-the-box in order to find solutions for logistical challenges.
- PSR can utilize its Orphan Toolkit to decrease the patient's and family's burden related to their clinical trial participation.



Wilson's Disease

Wilson's Disease is a rare genetic disorder characterized by excess copper stored in various body tissues, particularly the liver, brain, and corneas of the eyes. The disease is progressive and, if left untreated, it may cause liver (hepatic) disease, central nervous system dysfunction, and death.



PSR Orphan Experts: The leading expert in assisting biotech and pharma companies with orphan drug development and part of the Ergomed Group, which offers a full range of high-quality clinical research and trial management services.

Our dedicated team is looking forward to supporting you with your trial! Contact us today at info@psr-group.com

