

THE SUMMER ISSUE

THE LATEST FROM PTEN RESEARCH

We hope that everyone is safe and well and our thoughts go out to all of our contacts across the world.

At PTEN Research it is our goal to improve the lives of the patients and their families living with PHTS (PTEN Hamartoma Tumour Syndrome) by funding research into better understanding of the condition, and into the development of potential future treatments.

Thank you for continuing to support us.

- The PTEN Research Team

If you would like to read more about our activities please visit our website or email us at contact@ptenresearch.org

OUR RESEARCH

At PTEN Research we fund projects with leading experts in the field of PHTS at institutions around the world. A condition of our funding is the publication of the work to ensure PHTS patients and other scientists benefit from the work which we fund. Further, our formal application and external review processes ensure we only fund work which we believe is of high scientific value.

If you would like to find out more about how we fund research or apply for a research grant, please [click here](#).

NEWS AND EVENTS

PTEN Research Foundation appoints two new Scientific Advisory Board Members

The Trustees are pleased to announce the appointment of Dr Ian Waddell and Dr Leticia Toledo-Sherman as members of the Scientific Advisory Board to the PTEN Research Foundation.



Dr Waddell has over 30 years' experience in the pharmaceutical R&D sector and has worked in a variety of drug discovery settings. He has held various leadership positions at AstraZeneca over a period of almost 20 years. Other key positions held by Ian include head of biology in the Drug Discovery Unit at the Cancer Research UK Manchester Institute, and Chief Scientific Officer of early Discovery at the global Contract Research Organisation Charles River laboratories. Dr Waddell is also currently the Chief Scientific Officer at CUMULUS Oncology, an Edinburgh based cancer focused accelerator suite.

Ian is a biochemist by training with extensive expertise in Oncology focused drug discovery. He gained a BSc and PhD in Biochemistry at the University of Dundee. After 2 post docs he returned to Dundee and spent 5 years as a lecturer in Molecular Medicine in the Department of Child Health at Ninewell's Hospital and Medical School

before joining Zeneca in 1993.



Leticia is the Vice President of Chemistry at MycRX, an oncology biotech company working on first-in-class small molecule drugs against the oncoprotein Myc. Leticia has over 20 years of drug discovery and development experience in oncology, Huntington's Disease, Alzheimer's Disease, and Related Dementias from her work in the pharmaceutical, biotechnology, and nonprofit sectors. She is an accomplished scientist and senior executive with a proven track record of discovering and advancing innovative therapies towards patients. Most recently, Leticia was the Senior Director of Drug Discovery and Early Drug Development for the Tau Consortium, where she led the progression of a large portfolio of therapeutic and biomarker programs toward clinical development for Alzheimer's Disease and Related Dementias. Prior to that, Leticia was Director of Medicinal Chemistry and Computer-Aided Drug Design for the Cure Huntington's Disease Initiative (CHDI), a foundation following a biotech model to develop therapeutics for Huntington's Disease. At CHDI, Leticia led multiple therapeutic programs that delivered clinical candidates through the IND stage. Prior to CHDI, Leticia held leadership positions in pharma and biotech (Lymphosign, MDS Proteomics, and Amgen) working in oncology.

Leticia received her PhD in Organic Chemistry from The State University of New York at Stony Brook and was a postdoctoral fellow at The Massachusetts Institute of Technology, and The Scripps Research Institute. Leticia has been a member of scientific advisory and steering committees for the Structural Genomics Consortium, The Chemical Probes Portal, and NIH's Illuminating the Druggable Genome. She holds an adjunct appointment at UCLA, Department of Neurology. She is currently a

Scientific Advisory Board member for Grove Biopharma and the PTEN Foundation.



PTEN UK & Ireland Patient Day

The PTEN UK & Ireland Patient Day took place on Saturday July 2nd in Abingdon, Oxfordshire. The Foundation had the pleasure to attend and present an update on our strategy and progress to develop treatments for PHTS. It was wonderful to meet so many members of the PHTS community and exchange ideas in person. Principal Investigators for several Foundation funded projects provided updates including Professor Marc Tischkowitz on the [PHTS Registry](#), Professor Mustafa Sahin on the results of the [PHTS everolimus trial](#) and Professor Tom Frazier on the development of a PHTS specific neurobehavioural evaluation tool. In addition, several other interesting scientific presentations described ongoing research initiatives relevant to PHTS. The foundation thanks PTEN UK&I and all participating families for a successful day.

[Click here to watch videos from the day](#)

[Click here to visit the PTEN UK & Ireland website](#)



UK PHTS Patient Registry

Recruitment to the PHTS Registry is progressing, and is supported by project coordinator Katerina Stroud who started in this role in April 2022. Many thanks to all those who have taken part so far. Participation does not require any hospital visits and can be done from the comfort of your home.

PTEN UK and Ireland together with the lead investigator Professor Marc Tischkowitz have made a YouTube video which we hope will answer many of the questions that patients and families have about taking part in the Registry.

More information is available on the project website or via email at add-tr.phts@nhs.net

[Click here to view the YouTube videos](#)

[Click here to visit the PHTS Registry website](#)

Everolimus Study Data

Professor Mustafa Sahin at Boston Children's Hospital, together with colleagues at Stanford University and the Cleveland Clinic have recently published the results from a clinical trial of everolimus in individuals with PHTS and neurocognitive symptoms.

The 6-month, phase II, randomized trial included 46 participants aged 5-45 years. The results show that everolimus is well tolerated. Although the primary efficacy endpoint was not met, several secondary efficacy

endpoints showed some improvement. PTEN Research Foundation are proud to have been able to provide financial support for the conduct of this study which is the first ever controlled trial in individuals with PHTS.

[Click here to access the published study results](#)



If you would like to make a donation to support our work
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RESEARCH**

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