MEDIA RELEASE

New research advance identifies drug targets for a brain blood vessel disease

Cerebral cavernous malformations (CCM) are the most common cause of stroke in young people. These malformations also known as Cavernomas are present in 0.1-0.5% of the population, with about 60% causing symptoms. Currently there is no drug treatment available for CCM.

However, as opposed to stroke in the elderly where no single genetic defect is implicated, in CCM defect in one of three genes (CCM1, CCM2 and CCM3) causes CCM disease. Correcting the consequences of these defects thus provides a plausible treatment, but hitherto how defects in the CCM genes cause disease has been unknown.

A new study published today in Nature (Zhou et al) by Dr. Zheng's group at Centenary Institute, Sydney Medical School and collaborators at Perelman School of Medicine, University of Pennsylvania, has identified abnormalities in the endothelial lining of brain blood vessels that are raised the possibility of a therapeutic intervention. To test this possibility they used a genetically engineered mouse model to mimic human disease, where they were able to inhibit the expression of the culprit proteins and found they could prevent the formation of CCM and death from the subsequent strokes. This study provides three 'druggable targets' for the treatment for CCM disease.

Dr. Zheng has a long term involvement in understanding the genes causing CCM disease. The breakthrough for this study is based on their long time effort in making mouse models to mimic human disease and the recent invention of new micro-CT technology that allows visualization and quantification of the cavernous malformations in mice. This study exemplifies high quality of basic research can lead to the design of drugs for "untreatable" diseases.

MEDIA CONTACT: Ashleigh Mullaly, Media & Communications Manager, 0418 274 428 or a.mullaly@centenary.org.au