

MEDIA RELEASE

15 December 2023

Research aims to transform haemophilia treatment

Professor Philip Hogg, researcher in the Centenary Institute's Centre for Cancer Innovations, has received a National Health and Medical Research Council (NHMRC) Investigator Grant to undertake research aimed at revolutionising the treatment of haemophilia A.

A rare genetic disorder, haemophilia A is characterised by a deficiency of an essential blood-clotting protein called Factor VIII (FVIII), which leads to bleeding episodes, most commonly into the joints. This can lead to chronic pain, joint deformity, reduced mobility and increased mortality.

The grant, worth \$2,953,040 over five years, will enable Professor Hogg and his team to investigate a recently discovered aspect of FVIII – differing structural forms of this blood protein, influenced by the presence or absence of essential chemical bonds called disulphide bonds.

Professor Hogg says that his research could make a big difference for people with haemophilia A with the research leading to the development of cheaper, safer and longer-lasting treatments.

"Current treatment options for haemophilia A involve replacing the missing FVIII through infusions or by using gene therapy. However, these treatment approaches are limited in their effectiveness because the FVIII protein is inherently toxic," said Professor Hogg.

"Commercial production of recombinant FVIII for replacement therapy is very expensive because the protein kills the cells it is produced in. Gene therapy to produce FVIII is promising but not durable as the liver cells that make the protein die over time and damage the liver."

Professor Hogg believes that he may be able to identify a form of FVIII – based on its particular disulphide bond makeup – that overcomes these limitations. He will test more than 100 forms of the blood protein to identify a variant that is effective but less harmful to cells and patients.

"By identifying those forms of FVIII that are less toxic, we hope to improve the management of haemophilia A, reduce bleeding episodes and enhance the overall quality of life for those affected by this condition," said Professor Hogg.

Professor Marc Pellegrini, Centenary Institute Executive Director, said the successful grant was an excellent outcome for an important project.

"Haemophilia A requires ongoing medical attention to help prevent or control bleeding and treatment regimes can be demanding and severely impact the daily lives of those affected by this disorder," said Professor Pellegrini.

"I congratulate Professor Hogg on his successful grant. This exciting and potentially lifechanging research could lead to improvements in treatments for haemophilia A that affects about 1 in every 5,000 males."

<u>NHMRC Investigator Grants</u> support research across the four pillars of health and medical research – biomedical, clinical, public health and health services. The scheme allows the highest-performing researchers to pursue important new research directions, to form collaborations, and foster innovative and creative research.

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Images:

Professor Philip Hogg https://drive.google.com/file/d/1-RHkIC-wa7duEV6iBeMN4kYaiicsHg9A/view?usp=sharing

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About the Centenary Institute

The Centenary Institute is a world-leading independent medical research institute, closely affiliated to the University of Sydney and the Royal Prince Alfred Hospital. Our research spans the critical areas of cancer, cardiovascular disease, rare diseases, inflammation, infectious diseases, healthy ageing and biomedical AI. Our strength lies in uncovering disease mechanisms and applying this knowledge to improve diagnostics and treatments for patients.

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