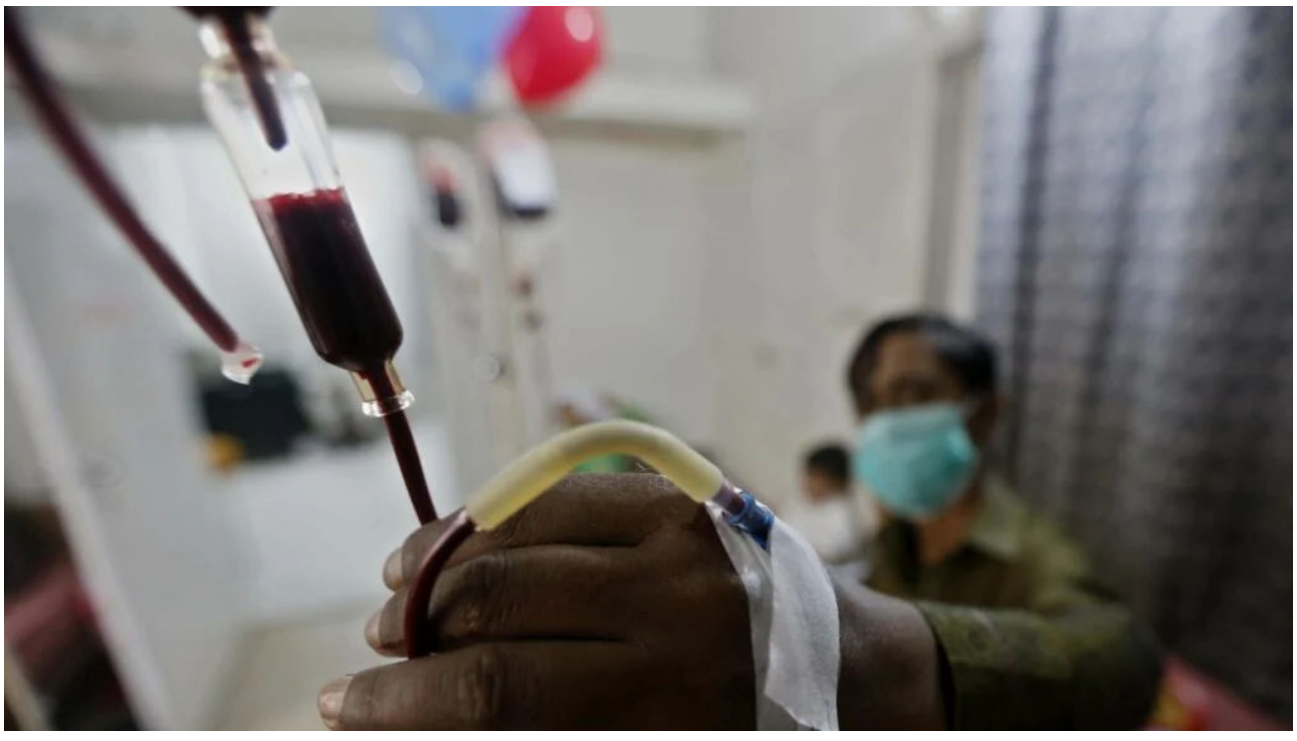
NEWS / AUSTRALIA

Research team lead by Dr Jim Vadolas develops viral gene therapy to treat beta thalassaemia

Thalassaemia is fatal if left untreated and patients are dependent on regular blood transfusions every three to four weeks for life



A thalassemia patient receives a blood transfusion at a medical centre. Photo: AAP via EPA/ARSHAD ARBAB

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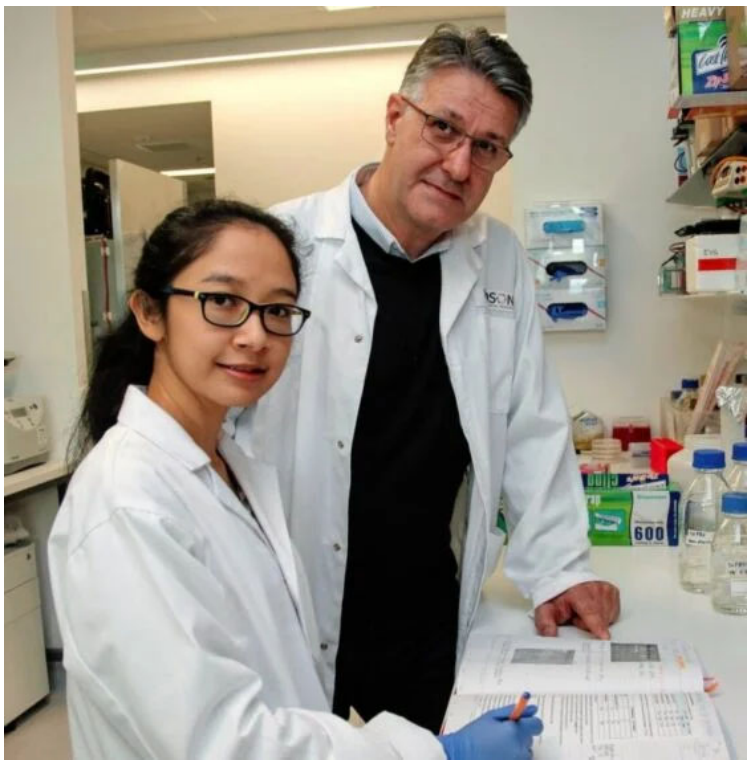
Immunohaematology researchers at the Hudson Institute led by Dr Jim Vadolas have developed a new gene therapy strategy to treat beta thalassaemia, an inherited disorder where the body doesn't make enough haemoglobin in red blood cells.

Using viral gene therapy – a process where therapeutic genes are delivered into blood stem cells by a virus – to both deliver the therapeutic beta globin gene and simultaneously limit the production of excess alpha globin the team found the lack of haemoglobin is caused by two issues – a mutation in the beta globin gene, and too much alpha globin, which is toxic to red blood cells.



(L-R): Dr Tiwa Nualkaew and Dr Jim Vadolas via Hudsaon.org.au

The goal of this research, conducted in the laboratory is to improve the quality of life and save lives by finding new treatments that improve haemoglobin production in beta thalassaemia patients many of whom come from Mediterranean, Middle Eastern, African and Asian populations.

The researchers' next step would be to deliver the therapy into patients after funding is secured.

"Our approach was guided by the knowledge that reduced production of excess alpha globin can moderate the beta thalassaemia disorder," said Dr Vadolas.

"Our therapeutic approach has only been possible by firstly understanding the causes of disease and secondly the development and refinement of sophisticated genetic tools able to be used in humans," said Dr Vadolas.

Australian Alumni Association of Cyprus presents the COVID-19 Australia-Cyprus Forum

The objective of the forum is to compare the course of the COVID-19 pandemic, the public health responses, and issues encountered in implementation of vaccination programs in Australia and Cyprus

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