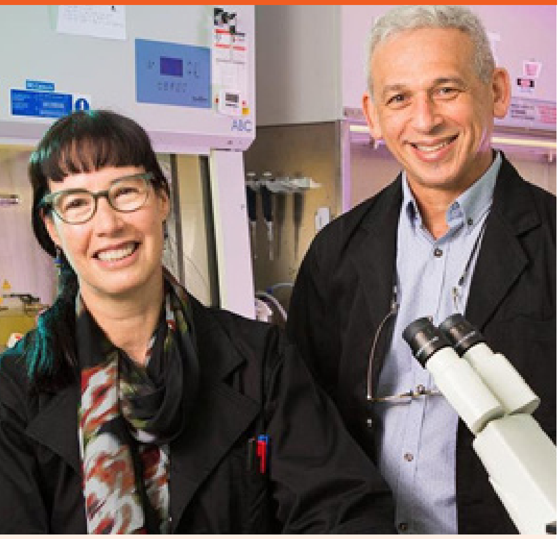
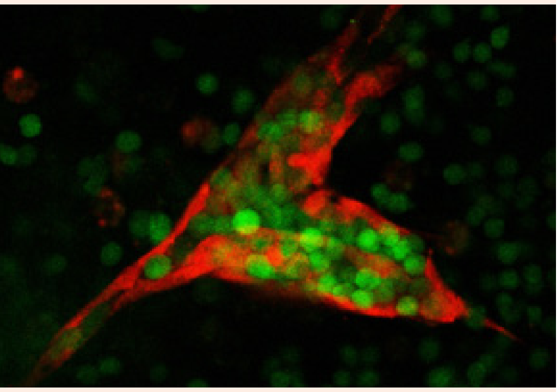
At a glance

A powerhouse trio of laboratories is refining the way of making blood and heart cells, bringing future therapies closer to reality.



Dr Elizabeth Ng and Professor Andrew Elefanty



Making the right cells from stem cells

The need

Pluripotent stem cells are an invaluable source of cells for research and for the development of new therapies for a range of diseases. Since pluripotent stem cells have the potential to make any cell in the body, a critical challenge is developing methods to coax stem cells to form the desired cell type, while ensuring they do not form unwanted cells. Perfecting these methods is necessary to enable pre-clinical research to advance into clinical trials, and eventually, new treatments.

The projects

The laboratories of Professor Andrew Elefanty, Professor Ed Stanley and Dr David Elliott are among the world's leaders in the art of making pluripotent stem cells turn into the cells that make up the blood, heart and their supporting tissues. The three teams are all based at the Murdoch Children's Research Institute, a co-location that facilitates the exchange of ideas and knowledge. The longstanding collaborations between these groups has helped advance critical research, underpinning the future use of stem cells for both clinical and biotechnological applications. So it's no surprise that these teams have also achieved significant breakthroughs in recent years.

In an international collaboration in 2011, Dr Elliott's team helped identify a new way to more easily obtain and purify heart muscle cells from stem cells, directly aiding efforts to study heart disease and develop new drug and cell therapies. More recently, his lab identified mutations in a gene called ALPK3 that prevents heart cells from correctly communicating with each other, resulting in a disease known as hypertrophic cardiomyopathy.

Building on years of research, the Stanley and Elefanty labs reported the successful conversion of human pluripotent stem cells to blood cells that closely resemble the first blood stem cells that are found during early human development. These cells provide researchers with an important new method for studying the development of blood disorders such as thalassemia and leukaemia.

The impact

Based on their extensive research achievements, the efforts of this trio of labs has far reaching impact. These range from the introduction of new genetic screens to identify and better support children and families at risk of developing hypertrophic cardiomyopathy, to advancing global efforts to develop suitable replacement heart cells for human transplantation. The capacity to develop laboratory-grown blood cells opens up the possibility of creating red and white blood cells for transfusion purposes, as well as regenerating bone marrow for patients with leukaemia and other blood diseases who lack a suitable matched donor. It is also anticipated that cells produced in the lab could be personalised for each individual, reducing the risks of graft rejection or graft-versus-host disease.