The Hon. Greg Hunt MP

Minister for Health

MEDIA RELEASE

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$69 million boost for fight against rare cancers and rare diseases

The Turnbull Government is supporting Australia's best and brightest medical researchers in their fight against rare cancers and rare diseases with a $69 million boost announced today.

This funding includes more than $26 million for nineteen research projects as part of the landmark Medical Research Future Fund's *Rare Cancers, Rare Diseases and Unmet Needs Clinical Trials Program*.

These projects will undertake clinical trials for devastating conditions like acute lymphoblastic leukaemia in infants, aplastic anaemia, multiple sclerosis and Huntington's disease.

This is a significant boost on the $13 million that was originally flagged when we called for applications and reflects the incredibly high calibre of medical research that is happening right here in Australia.

Researchers at the University of New South Wales will test a vaccine to target glioblastoma, a lethal brain cancer and the most frequent cause of cancer deaths in children and young people.

Another clinical trial at the University of Queensland will evaluate the benefits of medicinal cannabis for people with advanced cancer, and define the role of the drug for patients with cancer in palliative care.

Monash University is researching a new preventive treatment for graft versus host disease following a bone marrow transplant which could halve instances of the life-threatening complication, while a trial by the University of Western Australia to simultaneously compare a range of cystic fibrosis treatments may lead to improved care for this complex disease.

Other trials will explore the effectiveness and safety of aspirin compared to heparin to treat blood clots and test a new triple therapy regimen to target rare viral-driven brain lymphomas.

While we have seen improved survival rates for high incidence cancers such as bowel cancer, rates for rare cancers have remained relatively unchanged for some time.

In fact, rare cancers with low survival rates accounted for 47 per cent of all cancer deaths in 2014.

For people living with a rare disease and the medical professionals treating them, there are significant challenges including diagnostic delays, lack of available treatments and difficulty in finding the appropriate care.

We are committed to continuing to invest in research to find the answers to these challenges.

The overwhelming response to this Medical Research Future Fund (MRFF) clinical trial grant round demonstrates that there is plenty of research talent and enthusiasm to tackle rare cancers and rare diseases.

In recognition of this need and opportunity, the Turnbull Government will shortly open a targeted grant round worth $10 million for research into rare cancers and rare diseases with low survival rates.

And an additional $33 million worth of grants will be made available under the MRFF in 2018-19 to carry on this important work with a prioritised focus on rare cancers, rare diseases and unmet need.

I am also delighted to announce today the members of the Strategic Advisory Group which will support the $100 million Australian Brain Cancer Mission.

The Mission is a partnership between the Federal Government, philanthropists, medical experts, patients and their families.

It's aim is to double survival rates for people living with brain cancer over the next 10 years.

Members of the Strategic Advisory Group are Professor Adele Green AC, (Chair), Professor Douglas Hilton AO, Ms Sarah Mamalai, Mr Dustin Perry, Ms Robyn Leonard, Dr Chris Fraser, Professor Mark Rosenthal, A/Professor Rosalind Jeffree, Professor Grant McArthur, Professor Brandon Wainwright, Professor Andrew Scott AM, Ms Michelle Stewart, and Ms Michelle Burke.

The Turnbull Government recognises the importance of clinical trials to drive new ideas and achieve new discoveries that bring improvements to quality of life and survival rates.

Investing in health and medical research creates better health outcomes for Australians and the more-than $69 million announced today will help ensure our nation's strong reputation as a global leader in medical research continues.

(ENDS)

Rare cancer projects:

Researcher/ Institute/ Funding	**Project/ Summary (from researcher)**
Professor Maher Gandhi The University of Queensland $1,642,389	An Open label, Multicentre, Phase I study of Ibrutinib, Rituximab and EBV specific T-cells in Patients with EBV-positive Primary or Secondary CNS Lymphoma unsuitable for standard therapies. Although brain lymphomas are devastating, a number of innovative therapies are in clinical trials to try and improve outcomes. Unfortunately these trials exclude a rare subset of virus driven brain lymphomas that occur in the immunosuppressed for which no therapy exists. Based on our research, we propose an innovative triple therapy regimen that targets the unique biology of viral driven brain lymphomas which we believe will be effective and well-tolerated.
Doctor Rishi Kotecha Monash University $314,772	A collaborative study of the Interfant network (Australian sites): the feasibility, safety and efficacy of the addition of Blinatumomab to the Interfant-06 backbone in infants with MLL-rearranged Acute Lymphoblastic Leukaemia (The Blin-fant Study) Infants (<1 year of age) diagnosed with acute lymphoblastic leukaemia (ALL) is rare but devastating. Most have a distinct genetic change which makes them even more likely to die from their disease.

	This international study will examine if a novel drug, blinatumomab, can be safely added to the standard chemotherapy used to treat infants with ALL, and if it is better than chemotherapy alone. The results from this study will be used to develop the first worldwide trial for infant ALL.
Associate Professor Kerrie McDonald University of New South Wales $1,446,002	Immunotherapy Targeting of Cytomegalovirus antigens in Glioblastoma: INTERROGATE-GBM Glioblastoma (GBM) is uniformly lethal, and these tumours now represent the most frequent cause of cancer death in children and young adults. Current therapy is incapacitating and produces a median overall survival of <15 months because of limits defined by non-specific toxicity. We will clinically test a peptide vaccine that specifically targets patient GBM and redirects patients' own immune cells to recognise and destroy tumours.
Professor Andrew Scott La Trobe University $1,564,188	Prospective, multicentre trial evaluating FET-PET in high grade glioma This prospective multicentre trial will be the largest study performed to date, aiming to develop a novel imaging test (FET-PET) for the accurate evaluation of residual or recurrent disease in patients with high grade brain cancer. We also aim to establish the prognostic ability of FET-PET in patients with high grade glioma.
Associate Professor Andrew Wei Monash University $1,507,785	A registry-linked national platform trial to improve precision-based outcomes using novel therapies in acute myeloid leukaemia (AML) This proposal will create an integrated national clinical trial program aimed at improving outcomes for patients with AML through introduction of precision-based diagnosis, treatment and monitoring within the Australasian Leukaemia and Lymphoma Group. An adaptive 3-stage platform study will be established to validate novel target directed therapies. Within the framework of a national AML Registry, new technologies will be used to identify the genomic architecture within each leukaemia and track.

Rare diseases projects:

Researcher/ Institute/ Funding	**Project/ Summary (from researcher)**
Professor Martin Delatycki Murdoch Childrens Research Institute $1,227,418	The efficacy of rehabilitation for hereditary ataxias- a randomised controlled trial The hereditary cerebellar ataxias (HCAs) result in worsening incoordination and loss of the ability to walk. Many reduce lifespan. There are no medications proven to improve symptoms for most HCAs. We have shown some evidence of benefit from rehabilitation to improve symptoms of HCAs and here propose a larger study to definitively answer the question of whether rehabilitation does indeed improve the ability of individuals with HCAs to perform basic tasks required to live independently.
Professor William Hague The University of Adelaide $1,191,769	Treatment of Severe Early Onset Intrahepatic Cholestasis of Pregnancy Severe early onset intrahepatic cholestasis of pregnancy, a rare disorder, associated with itching and increased concentrations of serum bile acids, has increased risks of stillbirth, fetal anoxia and compromise, pre-term birth, pre-eclampsia and gestational diabetes. Treatment is not well established: we will test ursodeoxycholic acid vs rifampicin. There are few long term data on the offspring health.
Associate Professor Clement Loy University of Sydney $1,905,227	A randomised controlled trial, of N-Acetyl Cysteine, for premanifest Huntington gene expansion carriers (NAC-preHD) NAC-preHD is a clinical trial for people who are Huntington Disease (HD) genetic expansion carriers, who have not yet developed clinical manifestations. Participants will be randomly allocated either to an oral nutritional supplement N-Acetylcysteine or placebo, assessed clinically and using brain imaging, over 3 years. This will be the largest clinical trial for premanifest HD expansion carrier in the world and if found to be effective, can be rapidly implemented in the community.

Professor Anne-Louise Ponsonby Australian National University $887,072	A randomised placebo-controlled trial of combined mitochondrial agents for the treatment of fatigue and depression in multiple sclerosis with an assessment of the impact on kynurenine pathway metabolomics Recent work implicates mitochondrial function problems as determinants of brain damage and symptoms in multiple sclerosis. Mitochondria are the powerhouses of brain cells and they are very vulnerable to oxidant damage. Specific antioxidant regimens can rescue damaged mitochondria. This clinical trial will evaluate how a newly developed Australian combined mitochondrial therapy alleviates fatigue and depression among people with relapsing remitting multiple sclerosis and fatigue.
Associate Professor Thomas Snelling University of Western Australia $3,545,905	BEAT-CF: Bayesian Evidence-Adaptive Trial to optimise management of Cystic Fibrosis For rare diseases like CF, there is an urgent need to know which treatments work, which don't, and in whom. Most trials only compare two treatments at a time, assigning a fixed number of patients to each option even when evidence is accumulating that one is better than the other. We will simultaneously evaluate a range of CF treatments, progressively eliminating those found to be worse than available alternatives. We expect to show this approach can efficiently improve care for complex diseases.
Associate Professor Adam Vogel University of Melbourne $498,627	SpeechAtax: A rater-blinded randomised controlled trial of intensive home-based speech treatment for ataxia Progressive brain disorders often lead to profound difficulties speaking. No medical treatments are known to reverse the effects of neurodegeneration. Patients are desperate for an evidenced based treatment to reverse the effects of decline. We aim to evaluate the effectiveness of intensive, home-based rehabilitation using biofeedback for improving speech in adults with cerebellar disease. Outcomes will be immediately available to patients and clinicians.
Professor Claire Wainwright The University of Queensland $2,091,178	A platform clinical trial approach to the management of Mycobacterium abscessus complex (MABSC) Mycobacterium abscessus complex are multi-drug resistant organisms that are now seen more frequently and can result in severe lung infection in vulnerable individuals. There is no current evidence base on which to determine management. Treatment regimens that are currently used are complex, expensive and are often very poorly tolerated and outcomes are variable. This application seeks to set up a platform trial that will provide evidence on which to base management in the future.
Associate Professor Erica Wood Monash University $1,750,726	The DIAAMOND study: Diagnosis of aplastic anaemia, management, and outcomes utilising a national dataset Aplastic anaemia (AA) is a bone marrow disorder leading to profound anaemia, low platelet counts (risk of major bleeding) and low white blood cell counts (risk of serious infection). Mortality is as bad as many cancers. Better diagnosis and treatment is needed. This trial of a new agent, avatrombopag, which stimulates blood cell production, along with bone marrow laboratory studies and comprehensive genomics assessments, will help better understand and treat this life-threatening condition.

Unmet need projects:

Researcher/ Institute/ Funding	**Project/ Summary (from researcher)**
Professor Steven Chadban University of Sydney $1,117,150	The BEST-Fluids study: Better Evidence for Selecting Transplant Fluids End-stage kidney disease (ESKD) is a major health problem worldwide. Kidney transplantation is the best treatment, however not all kidney transplants work well. At the time of kidney transplantation, patients receive fluid through a drip and this fluid may affect how well the kidney works.

	The BEST-Fluids study will determine which fluid (Plasmalyte or normal saline) produces the best results, particularly how long the transplant takes to work well and how this affects long term survival.
Associate Professor David Curtis Monash University $1,570,198	CAST – A Randomised Phase 3 Trial of Cyclophosphamide after Sibling Allogeneic Haematopoietic Stem Cell Transplant Bone marrow transplant is an important curative treatment for patients with blood cancers. Unfortunately, 40% of patients will develop a life-threatening complication called graft versus host disease (GVHD). In this study, we will compare two strategies to prevent GVHD – the standard drugs used for almost 30 years and a new treatment. We predict that this new treatment will halve the risk of serious GVHD, leading to improved survival, quality of life and reduced health costs to the community.
Professor Stephen Davis University of Melbourne $1,285,820	STOP-MSU: Stopping haemorrhage with Tranexamic acid Ommenced Prehospital in a Mobile Stroke Unit A minority of stroke patients (15%) have intracerebral haemorrhage (ICH) but it is associated with a higher mortality and worse outcomes than ischemic stroke. STOP-MSU will be a Phase II trial of 50 patients, recruited < 1 hour from onset, based on non-contrast CT showing ICH, but not requiring demonstration of the spot sign. Patients will be randomized 1:1 to Tranexamic acid or placebo. The primary outcome will be reduction of hematoma growth from ambulance to the 24 hr follow-up scan.
Professor Janet Hardy The University of Queensland $1,363,040	Medicinal Cannabinoids to Relieve Symptom Burden in the Palliative Care of Patients with Advanced Cancer Medicinal cannabis has proven helpful for symptom relief in a few chronic diseases, but there is limited evidence regarding the benefits and safety for patients with advanced cancer. We will conduct the first clinical trial to rigorously evaluate the efficacy, safety and acceptability of medicinal cannabinoids for symptom relief in advanced cancer patients. The study will define the role of medicinal cannabis in the care of patients with cancer undergoing palliative care.
Professor Ian Harris University of New South Wales $934,848	CRISTAL: Cluster Randomised Trial of Aspirin versus Low molecular weight heparin for venous thromboembolism prophylaxis in joint replacement surgery, a registry-nested study Hip and knee replacement surgery may be complicated by blood clots in the leg or lung. Due to a lack of evidence, there is uncertainty about the role of aspirin in preventing clots, compared to the most common drug (heparin). There is considerable variation in practice in Australia. This study will use patients recruited to the National Joint Replacement Registry to test the effectiveness and safety of (cheaper) aspirin tablets in preventing clots compared to (more expensive) heparin injections.
Professor David Pilcher Monash University $753,355	The BLENDER Trial – Blend to Limit Oxygen in ECMO: A randomised Controlled Registry Trial The sickest patients with heart & lung failure sometimes require extracorporeal membrane oxygenation (ECMO). ECMO pumps blood into the body with very high oxygen levels. High oxygen levels may be harmful. A more conservative oxygen level is possible. We will randomly allocate 286 ECMO patients to a high or conservative oxygen target and measure improvement in patient outcomes. If effective this therapy will improve Australian lives, transform clinical practice, and yield major savings.