CIRM Disease Team Planning Award

Grant Award Details

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Grant Type: Disease Team Planning

Grant Number: DT1-00697

Investigator:

Name:	Mark Walters
Institution:	Children's Hospital of Oakland Research Institute
Type:	PI

Award Value: $16,939

Status: Closed

Grant Application Details

Application Title: CIRM Disease Team Planning Award

Public Abstract:

Children born with sickle cell anemia (SCA), caused by a genetic defect in hemoglobin, have severe anemia and damage to virtually all the body organs: the damage begins in infancy, and is frequently fatal by early adulthood. This is one of the most common inherited diseases in the world: because of California's ethnic diversity it is relatively common here, often in underserved populations. Our research team is dedicated to the treatment and, when possible, cure of this devastating disease. We and others have shown that transplantation of blood stem cells from bone marrow can cure SCA, and this type of stem cell therapy also is used in treating other blood and genetic diseases. Unfortunately, not all individuals are cured after bone marrow transplantation, as this is a risky treatment. We have carried out pioneering work showing that blood from the umbilical cord ("cord blood") of a newborn sibling can be used to cure blood disease in the affected sibling. A significant part of our effort is devoted to discovering ways to improve and extend the use of cord blood for blood cell transplantation and make this treatment less risky. Most recently, we have found that the placenta itself is a rich source of blood stem cells, and possibly also of stem cells for tissues other than blood. We have developed a program of clinical research that is closely associated and integrated with lab research that supports investigations into ways to improve stem cell transplantation and make it available to more people.

We propose to use support from CIRM to organize a planning group of scientists who conduct clinical and preclinical research to focus on new methods to improve blood stem cell transplantation for SCA. This investigative team will plan therapies using stem cell sources that include umbilical cord blood, placenta, bone marrow and embryonic stem cells. We will conduct a clinical trial to determine if it is safe and effective to perform transplantation with purified hematopoietic stem cells. We will explore new methods to increase yields of cord blood stem cells after collection, so that cord blood transplantation can be made available to a broader population of recipients. We will also investigate and develop the use of placentally derived blood stem cells in transplantation, which may in the next few years extend this type of therapy to many more individuals who currently need it but do not have suitable stem cells available. We will also investigate treatments that will take longer to develop, including research that will study if it is possible to expand and then purify a population of immune cells from embryonic stem cells, and eventually improve the safety of unrelated and mismatched donor transplantation. This planning group is devoted to extending the use of blood stem cell transplantation for genetic diseases in California, and is very likely to contribute significantly to CIRM's goal of providing new cures for human disease.

Statement of Benefit to California:	The inherited blood disease sickle cell anemia afflict significant numbers of individuals in California, particularly in underserved populations; affected individuals require expensive treatment, and experience severe tissue damage that degrades quality of life and is often fatal. Individuals afflicted by sickle cell anemia can be cured by transplantation of blood stem cells. Nevertheless the application of this treatment is limited because the supply of donated bone marrow stem cells is limited, and because it is necessary to match certain characteristics of the donor's and host's immune systems. In order to expand the application of transplantation for sickle cell anemia, we will assemble a team of clinical and basic scientists from California biomedical institutions with the goal of developing and conducting clinical trials of novel stem cell therapies for sickle cell anemia. We will use support of the planning group to formulate a feasible path to carry out research dedicated to the improvement of methods that use stem cells from cord blood, placenta, bone marrow and embryonic stem cells for blood stem cell transplantation. The purpose of this planning group is to develop a final application that will develop collaboration and methods that directly support the proposed transplantation studies, with the aim that these may produce advances that will allow many more people to receive this curative therapy. In the course of this research, individuals with inherited blood diseases will receive transplants, and our extensive experience indicates these will be curative in the large majority of cases. The enhanced and extended lives of these individuals will represent a direct benefit; the savings to the health care system as a consequence of their cure is less direct but will benefit all California taxpayers. Ultimately the knowledge and experience produced by the research will contribute to the goal of making blood stem cell transplantation available to a much broader group of patients, thus greatly extending the benefits to the affected individuals and to the taxpayers of California.