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**Airways for Children**

**Grant Award Details**

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Airways for Children

**Grant Type:** Disease Team Therapy Planning I

**Grant Number:** DR2-05298

**Investigator:**

<b>Name:</b>	Martin Birchall
<b>Institution:</b>	University of California, Davis
<b>Type:</b>	PI

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**Disease Focus:** Respiratory Disorders

**Award Value:** \$19,800

**Status:** Closed

**Progress Reports**

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**Reporting Period:** Year 1

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**Grant Application Details**

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**Application Title:** Airways for Children

**Public Abstract:**

The primary goal is to bring a safe and effective therapy to children with severe large airway disease. Our intent is to implement all of the necessary steps for a successful new stem/progenitor cell-derived airway transplant for clinical trials in children within 4 years. Our team builds on first-in-human surgical success with a stem cell-based tissue engineered airway implant in a compassionate care case in a young adult and in a child. To this end, we will perform the necessary preclinical studies to support a successful FDA application within 4 years. We propose to use stem/progenitor cells from the patient to treat an extraordinarily difficult-to-manage health problem in children, namely large airway disease. In children this leads to collapse of tracheal cartilage causing severe airway obstruction that is life-threatening. It occurs in approximately 200 California children each year and the morbidity and mortality associated with this disease is very high. Approximately 25% of these young patients die before their first birthday. Treatment costs for these children are very high, and the familial and societal investments are substantially higher, although outcomes are consistently poor. The endpoint desired is normal airway and lung function and a normal quality of life. Our team aims to eliminate the need for repeated surgical interventions which are not necessarily successful, presently the standard of care for children with large airway obstruction. Bioengineered airway transplants that use the cells of the patients could be used in humans of all age groups and would not require lifelong, harmful anti-rejection medications.

In 2008 and 2010, a stem cell based, tissue engineered tracheal implant was successfully used by our team to save a young woman's and a child's life, respectively. These first-in-human studies emphasize that our goal is realistic and paves the way for clinical trials in children after carefully designed safety studies are completed for preclinical testing. Stem/progenitor cell-derived airway transplants that use the patient's cells have the clinical advantage of not requiring anti-rejection medications long-term. Our experience, to date, indicates such medication is not needed and this finding represents a scientific and clinical breakthrough in organ transplantation. While clear medical benefit was demonstrated in these proof-of-principle, compassionate care human cases, there is substantial work that must be done before considering such transplants for pediatric patients, and on a large scale, for adults. We address this challenge with our team approach and emphasize the synergism resulting from linking team members with expertise in a variety of scientific and medical disciplines to address this critical need. This new therapeutic approach could offer a tremendous benefit to children and patients in other age groups in the State of California that are in desperate need of new treatment options.

**Statement of Benefit to California:**

The citizens of California have generously invested in stem cell research and a return on their investment will include breakthroughs in medical treatments for diseases where there are currently limited options. Stem/progenitor cell-derived airway transplantation is a leading example of translational research in regenerative medicine that can be used for a host of diseases. Through this team effort scientists and physicians will lead the early promise of airway transplantation to clinical trials in California and beyond.

The research team proposes to use stem and progenitor cells to cure an extraordinarily difficult to manage and life-threatening health problem in children. Severe airway obstruction occurs in approximately 200 California children each year. The morbidity and mortality associated with this disease is very high; approximately 25% of patients will die before their first birthday. The knowledge gained from the preclinical studies proposed will provide a new technology that can be applied to other disorders in California populations. We foresee that our stem cell-derived airway transplant could also be applied to treat an important subpopulation of adults with severe chronic obstructive pulmonary disease (COPD) and the large number of children and adults with severe subglottic stenoses that have proven refractory to standard surgical interventions, and patients with debilitating laryngeal scarring. Given that the prevalence rate of COPD for California citizens greater than 65 years of age approaches 10%, if even 0.1% of COPD patients in California were candidates, specifically those with associated tracheobronchomalacia, then greater than 3,000 patients might benefit from this treatment. The methods and technology developed from this project can also be used as the basis for other similar health needs including esophageal, bladder, and bowel replacements for disorders where present treatments are very limited and impair quality of life.

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