FUNDING OPPORTUNITIES

FEDERAL FUNDING AGENCIES

National Institutes of Health

Advancing Novel Science in Women’s Health Research (ANSWRH) (R21) (PAS-10-226)
Office of Research on Women’s Health
John E. Fogarty International Center
National Center for Complementary and Alternative Medicine
National Cancer Institute
National Center for Minority Health and Health Disparities
National Eye Institute
National Human Genome Research Institute
National Heart, Lung, and Blood Institute
National Institute on Aging
National Institute on Alcohol Abuse and Alcoholism
National Institute of Allergy and Infectious Diseases
National Institute of Arthritis and Musculoskeletal and Skin Diseases
National Institute of Biomedical Imaging and Bioengineering
Eunice Kennedy Shriver National Institute of Child Health and Human Development
National Institute on Drug Abuse
National Institute on Deafness and Other Communication Disorders
National Institute of Dental and Craniofacial Research
National Institute of Diabetes and Digestive and Kidney Diseases
National Institute of Environmental Health Sciences
National Institute of General Medical Sciences
National Institute of Mental Health
National Institute of Neurological Disorders and Stroke
National Institute of Nursing Research
National Library of Medicine
Office of Behavioral and Social Science Research
Office of Dietary Supplements
applications

Epigenomics of Human Health and Disease (R01) (RFA-ES-10-002)
National Institute of Environmental Health Sciences
National Center for Complementary and Alternative Medicine
National Cancer Institute
National Heart, Lung, and Blood Institute
National Institute on Aging
National Institute on Alcohol Abuse and Alcoholism
National Institute of Arthritis and Musculoskeletal and Skin Diseases
Eunice Kennedy Shriver National Institute of Child Health and Human Development
National Institute on Drug Abuse
National Institute of Diabetes and Digestive and Kidney Diseases
National Institute of Neurological Disorders and Stroke
National Institute of Nursing Research
Office of Research on Women's Health
Application Receipt Date(s): September 29, 2010

Epigenomic Modifications in Neurodevelopment (R01) (RFA-MH-11-030)
National Institute of Mental Health
Application Receipt Date(s): October 20, 2010

Epigenomic Modifications in Neurodevelopment (R21) (RFA-MH-11-031)
National Institute of Mental Health
Application Receipt Date(s): October 20, 2010

2011 NIH Directors Pioneer Award Program (DP1) (RFA-RM-10-008)
NIH Roadmap Initiatives
Application Receipt Date(s): September 13, 2010

2011 NIH Directors New Innovator Award Program (DP2) (RFA-RM-10-009)
NIH Roadmap Initiatives
Application Receipt Date(s): September 20, 2010

Clinical Studies of Safety and Effectiveness of Orphan Products Research Project Grant (R01) (RFA-FD-11-001)
Food and Drug Administration
Office of Orphan Products Development
Application Receipt Date(s): February 2, 2011; February 1, 2012 Resubmission Due Date(s):
October 14, 2011 and October 15, 2012

Neurological Sciences Academic Development Award (K12) (PAR-10-229)
National Institute of Neurological Disorders and Stroke
Application Receipt Date(s): October 12, 2010; October 12, 2011 for new applications November 12, 2010; November 12, 2011 for resubmission and renewal applications AIDS Application Due Date(s):
January 7, 2011; January 7, 2012

Innovative Therapies and Tools for Screenable Disorders in Newborns (R01) (PAR-10-230)
Eunice Kennedy Shriver National Institute of Child Health and Human Development
The document contains information about various grants and funding opportunities from different federal agencies. Here is a structured representation of the key points:

### Innovative Therapies and Tools for Screenable Disorders in Newborns (R03) (PAR-10-231)
- **Eunice Kennedy Shriver National Institute of Child Health and Human Development**
- Application Receipt/Submission Date(s): Multiple dates, see announcement.

### Innovative Therapies and Tools for Screenable Disorders in Newborns (R21) (PAR-10-232)
- **Eunice Kennedy Shriver National Institute of Child Health and Human Development**
- National Institute on Deafness and Other Communication Disorders
- National Institute of Diabetes and Digestive and Kidney Diseases
- Application Receipt/Submission Date(s): Multiple dates, see announcement.

### National Institute of Biomedical Imaging and Bioengineering Program Project (P01) Applications (PAR-10-233)
- National Institute of Biomedical Imaging and Bioengineering
- Application Receipt/Submission Date(s): May 25, 2013

### Bioengineering Research Partnerships (BRP)[R01] (PAR-10-234)
- National Institute of Biomedical Imaging and Bioengineering
- National Cancer Institute
- National Eye Institute
- National Human Genome Research Institute
- National Heart, Lung, and Blood Institute
- National Institute on Aging
- National Institute on Alcohol Abuse and Alcoholism
- **Eunice Kennedy Shriver National Institute of Child Health and Human Development**
- National Institute on Drug Abuse
- National Institute on Deafness and Other Communication Disorders
- National Institute of Dental and Craniofacial Research
- National Institute of Environmental Health Sciences
- National Institute of Neurological Disorders and Stroke
- Application Receipt/Submission Date(s): Multiple dates, see announcement.

### NON-FEDERAL FUNDING AGENCIES

**New York Stem Cell Foundation Launches Innovator Awards Program for Early Career Investigators**

Grants of up to $1.5 million over a period of five years will be awarded to early career investigators working in translational stem cell research.

Posted on June 28, 2010
Deadline: August 27, 2010
The New York Stem Cell Foundation, which has supported more than twenty-three outstanding young stem cell scientists through its postdoctoral fellowship program, has announced a significant expansion of its efforts to cultivate the next generation of stem cell scientists.

The foundation’s recently announced Innovator Awards Program for Early Career Investigators will provide a total of $24.5 million in funding to seventeen investigators (up to $1.5 million each over five years) to launch their own laboratories, train other scientists, and foster innovative high-risk/high-reward research to explore the therapeutic potential of stem cells derived from humans and model organisms.

The program is designed to support scientists in the next phase of their career after postdoctoral work. The foundation has issued a Request for Applications to scientists performing translational stem cell work.

To be eligible, candidates must have an M.D., Ph.D., or an equivalent degree; have completed postdoctoral training; have demonstrated the ability to independently supervise staff and research; and have shown true innovation for translation of basic science to a clinical setting. International investigators may apply, but research must be conducted in the United States.

In addition to funding, Early Career Investigators will have access to the NYSCF’s laboratory and resources and the opportunity to present at the annual NYSCF conference.

The RFA is available at the NYSCF Web site.

Contact:  [Link to Complete RFP]

Epilepsy Foundation

Research Grants Program
Application Deadline: August 31, 2010
Award Amount: $50,000/year (maximum $100,000/two years)
Program guidelines
Apply via Proposal Central website
Proposal Central website tip: filter the list to show Epilepsy Foundation programs

The Research Grants Program stimulates epilepsy research by providing funding for investigators in the early stages of their careers. Seed grants are awarded to clinical investigators or basic scientists for support of biological or behavioral research which will advance the understanding, treatment, and prevention of epilepsy. Applications from established investigators (Associate Professor level or above) and those that are PIs or co-investigators on major federal grants (e.g. RO1 from NIH) are ineligible.

Predoctoral Research Training Fellowship
Application Deadline: August 31, 2010
Award Amount: $20,000/year
Program guidelines
Apply via Proposal Central website
Proposal Central website tip: filter the list to show Epilepsy Foundation programs

The Predoctoral Research Training Fellowship supports pre-doctoral students with dissertation research related to epilepsy, thus strengthening their interest in establishing epilepsy research as a career direction. Graduate students must be matriculating in a full-time doctoral (Ph.D.) program with an academic career focus. Areas of interest considered include, but are not limited to
neuroscience, physiology, pharmacology, psychology, biochemistry, genetics, nursing, or pharmacy may apply. ($20,000/year)

**Postdoctoral Research Fellowship**
Application Deadline: August 31, 2010
Award Amount: $45,000/year

**Program guidelines**
**Apply via Proposal Central website**

**Proposal Central website tip:** filter the list to show Epilepsy Foundation programs

The Postdoctoral Research Fellowship develops academic physicians and scientists committed to research related to epilepsy. Applications are considered equally from individuals interested in acquiring experience either in basic laboratory research or in the conduct of human clinical studies. Physicians or Ph.D. neuroscientists who desire postdoctoral research experience are eligible to apply. ($45,000/year)

**Research and Training Fellowships for Clinicians**
Application Deadline: October 1, 2010
Award Amount: $50,000/year (maximum $100,000/two years)

**Program guidelines**
**Apply via Proposal Central website**

**Proposal Central website tip:** filter the list to show Epilepsy Foundation programs

The Research and Training Fellowships for Clinicians is for clinically trained professionals. This fellowship is one-year of supervised study and research. Individuals with an MD or DO who will have completed residency training in neurology, neurosurgery, pediatrics, internal medicine, or psychiatry by the time the fellowship commences may apply. Other clinically trained professionals (i.e. PharmD, Doctor of Nursing) are also eligible to apply. ($50,000/year, up to 2 years)

**TSC PUBLICATIONS**


Curatolo P, Moavero R (2010) *Can we change the course of epilepsy in tuberous sclerosis complex?* Epilepsia 51(7):1330-1


CONFERENCES, MEETINGS AND SEMINARS

September 12-13, 2010
Epilepsy and Depressive Disorders
The Westin Chicago River North Hotel, Chicago, IL
Abstract deadline: March 26, 2010
www.epilepsyanddepressivedisorders.com

September 18-22, 2010
European Respiratory Society
Barcelona, Spain
www.erscongress2010.org

September 23-24, 2010
13th Annual NCHPEG Meeting: Genetics and Public Health
Hyatt Regency Bethesda, Maryland
http://www.nchpeg.org/index.php?option=com_content&view=article&id=110&Itemid=112

September 23-24, 2010
‘PKD - More Than a Kidney Disease’
Wellcome Conference Centre, Euston Rd, London, UK
Program information can be found by going to www.pkdcharity.co.uk/pkdconference.html. If you have further questions, please contact Tess Harris, Chairman, PKD Charity, at tessharris@pkdcharity.org.uk.

October 1-3, 2010
Epileptology: Comprehensive Review and Practical Exercises
InterContinental Hotel and Bank of America Conference Center, Cleveland, OH
www.cccfme.org/epilepsy10
October 3-6, 2010
Epilepsy Surgery: Invasive Evaluation
InterContinental Hotel and Bank of America Conference Center, Cleveland, OH
www.ccfcme.org/epilepsy10

October 20-22, 2010
4th Biomarker Discovery and Development Conference
San Francisco, CA
http://gtcbio.com/conferenceDetails.aspx?id=154

October 23-25, 2010
13th Society for the Study of Behavioral Phenotypes (SSBP) International Research Symposium: Neurobehavioural Variability in Genetic Disorders
Neurological Institute, IRCCS C. Mondino Foundation, Pavia, Italy
For more information: http://www.ssbp.co.uk

November 11-12, 2010
9th Annual Molecular and Cellular Cognition Society Meeting
San Diego, CA
For more information: http://www.molcellcog.org

March 16-19, 2011
Third International Chordoma Research Conference
Bethesda North Marriott, Bethesda, MD
For more information: Click here to RSVP and register

July 6-9, 2011
Summit on Drug Discovery for TSC & Related Disorders
Omni Shoreham Hotel, Washington, DC
More information coming soon at www.tsalliance.org

September 22-24, 2011
International TSC Research Conference
Belfast, Northern Ireland
More information coming soon!

NEWS

Call for Nominations for Manuel R. Gomez Award

Nominations are being accepted until September 1, 2010 for the 2010 Manuel R. Gomez Award. This award is given in memory of Manuel R. Gomez, MD, the “Father of TSC” in the USA. Dr. Gomez provided care and compassion for hundreds of individuals with TSC during his career, and sparked interest in the diagnosis and clinical care of individuals with TSC. Individuals may nominate individuals who have either made a significant impact on our understanding of TSC in research and/or who have provided exceptional clinical care for individuals with TSC. Send a letter of nomination and a current CV for the nominee by no later than September 1, 2010 to Katie Smith, Science Coordinator, TS Alliance, 801 Roeder Road, Suite 750, Silver Spring, MD or ksmith@tsalliance.org

Previous recipients of the Manuel R. Gomez Award include:
- 2009 – Sergiusz Jozwiak, M.D., Ph.D., Memorial Children’s Hospital, Warsaw, Poland
- 2008 – Elizabeth Thiele, M.D., Ph.D., Massachusetts General Hospital, Boston, MA
- 2007 – Ann Hunt, Tuberous Sclerosis Association, Oxfordshire, UK
- 2006 – David Neal Franz, M.D., Children’s Hospital Medical Center, Cincinnati, OH
RESEARCH RESOURCES

New Resource for Drug Developers: The Rare Disease Repurposing Database (RDRD)
The U.S. Food and Drug Administration’s Office of Orphan Products Development (OOPD) has established a new resource for drug developers—a database of products that:
- have received orphan status designation (i.e. they’ve been found “promising” for treating a rare disease) AND;
- are already market-approved for the treatment of some other diseases.

For more information, go to: http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/ucm216147.htm

TSC1 and TSC2 Variation Databases
The TS Alliance funded the development and ongoing curation of the TSC1 and TSC2 Variation Database. These databases contain the reported variations (mutations and polymorphisms) in the TSC1 and TSC2 genes. The database is curated by Rosemary Ekong, PhD and Sue Povey, PhD at the University College London. To access the databases, go to:

TSC1 Database: http://chromium.liacs.nl/LOVD2/TSC/home.php?select_db=TSC1

TSC CLINICAL TRIALS

For a complete list of TSC clinical trials, go to: http://www.tsalliance.org/pages.aspx?content=370

Information can also be found at: http://www.clinicaltrials.gov

Efficacy and Safety of RAD001 in Patients Aged 18 and Over With Angiomyolipoma Associated With Either Tuberous Sclerosis Complex (TSC) or Sporadic Lymphangioleiomyomatosis (LAM) (EXIST-2)

Purpose: This study will evaluate the safety and efficacy of RAD001 in treating patients with Angiomyolipomas associated with Tuberous Sclerosis Complex or Sporadic Lymphangioleiomyomatosis.

Primary Outcome Measures:
- Angiomyolipoma response rate through MRIs at screening, 12, 24 and 48 weeks and annually thereafter [Time Frame: Screening, 12, 24, 48 weeks and annually for 4 years]

Secondary Outcome Measures:
- Time to angiomyolipoma progression through MRIs at screening, 12, 24 and 48 weeks and annually thereafter [Time Frame: Biomarkers assessed throughout the first year of the study only. All other outcomes are assessed through the duration of the study (5 years).]
• Skin lesion response rate tracked by digital photographs [Time Frame: Biomarkers assessed throughout the first year of the study only. All other outcomes are assessed through the duration of the study (5 years).]
• Change from baseline in biomarkers collected during the first years of study [Time Frame: Biomarkers assessed throughout the first year of the study only. All other outcomes are assessed through the duration of the study (5 years).]
• Changes in renal function by assessing creatinine clearance levels throughout the study [Time Frame: Biomarkers assessed throughout the first year of the study only. All other outcomes are assessed through the duration of the study (5 years).]
• Safety assessed on a continuous basis throughout study [Time Frame: Biomarkers assessed throughout the first year of the study only. All other outcomes are assessed through the duration of the study (5 years).]

**Estimated Enrollment:** 99 (40 study participants enrolled to date on July 28, 2010)

**Study Start Date:** March 2009

**Estimated Primary Completion Date:** January 2014 (Final data collection date for

<table>
<thead>
<tr>
<th>Arms</th>
<th>Assigned Interventions</th>
</tr>
</thead>
<tbody>
<tr>
<td>1: RAD001 5mg: Experimental</td>
<td>Drug: Everolimus (RAD001) 5 mg in tablet form. 10mg daily dosing throughout the trial.</td>
</tr>
<tr>
<td>2: RAD001 Placebo: Placebo Comparator</td>
<td>Other: Placebo Placebo</td>
</tr>
</tbody>
</table>

**Eligibility:**
Ages Eligible for Study: 18 Years and older
Genders Eligible for Study: Both
Accepts Health Volunteers: No

**Criteria for participating in study:**

**Inclusion Criteria:**
• 18 years or older
• Definite diagnosis of Tuberous Sclerosis or Sporadic Lymphangioleiomyomatosis (LAM)
• At least one Angiomyolipoma (AML) of at least 3 cm in diameter
• Females of child bearing potential must use birth control

**Exclusion Criteria:**
• Recent heart attack, cardiac related chest pain or stroke
• Severely impaired lung function
• Bleeding related to angiomyolipoma
• Severe liver dysfunction
• Severe kidney dysfunction
• Pregnancy or breast feeding
• Current infection
• History of organ transplant
- Surgery within two months prior to study enrollment
- Prior therapy with a medication in the same class as Everolimus
- Uncontrolled high cholesterol
- Uncontrolled diabetes
- HIV
- Patients with metal implants thus prohibiting MRI evaluations

Other protocol-defined inclusion/exclusion criteria may apply.

**Locations:**

University of Alabama at Birmingham  
BIRMINGHAM, Alabama, United States, 35294  
Contact: Christin Barger  205-934-9575  cbarger@uab.edu  
Principal Investigator: Bruce Korf, M.D.

Barrow Tuberous Sclerosis Center  
PHOENIX, Arizona, United States, 85013  
Contact: Georgann Vanderjagt  602-406-3825  georgann.vanderjagt@chw.edu  
Principal Investigator: Vinodh Narayanan, M.D.

Miami Children's Hospital  
MIAMI, Florida, United States, 33155  
Contact: Ricardo Luzondo  305-663-6802  ricardo.luzondo@mch.com  
Principal Investigator: Michael Duchowny, M.D.

Massachusetts General Hospital  
BOSTON, Massachusetts, United States, 02114  
Contact: Susana Camposano  617-726-0240  scamposano@partners.org  
Principal Investigator: Elizabeth Thiele, M.D.

Minnesota Epilepsy Group  
ST. PAUL, Minnesota, United States, 55102  
Contact: Lisa Albers  651-241-5058  lalbers@mnepilepsy.net  
Principal Investigator: Michael Frost, M.D.

Cincinnati Children's Hospital Medical Center  
CINCINNATI, Ohio, United States, 45229-3039  
Contact: Elizabeth Jo Coombs  513-636-6274  elizabeth.coombs@cchmc.org  
Principal Investigator: John Bissler, M.D.
Toronto General Hospital
Toronto, Ontario, Canada, M5G 2C4
Contact: Margaret Mcgrath-Hhong 416-340-4257 margaret.mcgrath-chong@uhn.on.ca
Contact: York Pei, M.D. 416-340-4800 ext 4257 york.pei@uhn.on.ca
Principal Investigator: York Pei, M.D.

More information about the Novartis sponsored TSC clinical trials can be found at:
http://www.novartisclinicaltrials.com/webapp/portals/EXISTClinicalTrials/page.do

INFORMATION ABOUT TSC

For information about TSC and the Tuberous Sclerosis Alliance, visit the TS Alliance Web site at:
http://www.tsalliance.org or contact the TS Alliance at info@tsalliance.org or by telephone: 1-800-225-6872 or 301-562-9890.

This is the August 2010 edition of TSC Alert – an online research newsletter for individuals interested in Tuberous Sclerosis Complex (TSC) research and clinical care. This online newsletter contains information of interest to the TSC research and health care community. Please forward this newsletter to colleagues who are interested in TSC. To be added/deleted to/from the mailing list for TSC Alert and/or to submit information for the September 2010 TSC Alert contact: vwhittemore@tsalliance.org

 Archived issues of the TSC Alert can be found at: http://www.tsalliance.org/pages.aspx?content=25