

## Curriculum Vitae

Date: 1/6/17

### A. Identifying data:

Name: Gregory Mark Enns, M.B., Ch.B.  
Ethnicity: Caucasian  
Citizenship: United States of America  
Licensure: California #A52095  
Hawaii #MD12592  
United Kingdom #3469202

### B. Academic History:

Undergraduate: B.A., Biology: May, 1984  
Pomona College  
Pomona, CA  
August, 1980–May, 1984

Graduate: Diploma, Medical Science: June, 1987  
University of St. Andrews  
St. Andrews, Scotland  
September, 1985–June, 1987

M.B., Ch.B.: June, 1990  
University of Glasgow  
Glasgow, Scotland  
September, 1987–June, 1990

Honors and Awards: Kellogg Foundation Scholarship, UC Davis, 1984  
P.T. Herring Memorial Prize in Histology, University of St. Andrews, 1986  
First Class Merit Certificates: Anatomy, Physiology, Biochemistry, General Pathology, Microbiology, and Pharmacology, University of St. Andrews, 1985-87  
Neil Arnott Memorial Prize in Clinical Physics, University of Glasgow, 1988  
University of Glasgow Clinical Travel Grant, for clinical studies in China and Nepal, 1988  
C.H.L.A. Board of Directors Award for outstanding service as Pediatric Chief Resident, 1995  
U.C.S.F. Liver Center Pilot/Feasibility Grant for Hepatic Gene Therapy Research, 1998  
Pete and Arline Harman Scholarship, 2005

Horizon Exhibit 2007

Stanford University School of Medicine Honor Roll for Teaching,  
2005-2006  
Distinguished Service Citation, American Academy of Pediatrics,  
2011  
Outstanding Service Citation, United Mitochondrial Disease  
Foundation, 2011  
Stanford University School of Medicine Excellence in Teaching  
Citation, 2011–2012  
PCARES Rose Award, Lucile Packard Children's Hospital, 2015

Post-Graduate Training: Junior House Officer, Pediatric Surgery  
Royal Hospital for Sick Children, Yorkhill  
Glasgow, Scotland  
August, 1990–January, 1991

Junior House Officer, General Medicine  
Glasgow Royal Infirmary  
Glasgow, Scotland  
January, 1991–June, 1991

Intern and Resident, Pediatrics  
Children's Hospital of Los Angeles  
Los Angeles, California  
June, 1991–June, 1994

Chief Resident, Pediatrics  
Children's Hospital of Los Angeles  
Los Angeles, California  
June, 1994–June, 1995

Fellow, Medical Genetics  
University of California, San Francisco  
San Francisco, California  
June, 1995 – June, 1998

Board Certification: American Board of Pediatrics (10/12/94, 6/14/01, 11/06/08)  
American Board of Medical Genetics, Diplomate, Clinical Genetics  
(9/1/99, 1/1/10)  
American Board of Medical Genetics, Diplomate, Clinical  
Biochemical Genetics (9/1/99, 1/1/10)  
ECFMG 0-447-053-0

Research/Contract  
Support:

- RP103-MITO-001 (Enns) 10/08/14–09/30/16 3.18 CM  
Raptor Pharmaceuticals \$254,794  
An Open-label, Dose-Escalating Study to Assess the Safety, Tolerability and Efficacy of Cysteamine Bitratrate Delayed-release capsules (RP103) for the Treatment of Children with Inherited Mitochondrial Disease  
*This is an open-label study focusing on the safety and preliminary efficacy of cysteamine bitatartrate to treat patients with Leigh syndrome and other inherited mitochondrial disorders.*
- RP103-MITO-002 (Enns) 07/20/15–05/31/17 1.66 CM  
Raptor Pharmaceuticals \$359,162  
A Long-Term Open-Label Extension Study of RP103-MITO-001 to Assess the Safety, Tolerability and Efficacy of Cysteamine Bitratrate Delayed-release capsules (RP103) for the Treatment of Children with Inherited Mitochondrial Disease  
*This is an extension study for the RP103 protocol, a study using cysteamine bitartrate to treat patients with Leigh syndrome and other inherited mitochondrial disorders.*
- IND#107,401 (Enns) 12/09/13 – 11/30/16 0.56 CM  
Edison Pharmaceuticals \$224,765  
Long-Term Safety and Efficacy Evaluation of EPI-743 in Children with Leigh Syndrome  
*This is a long-term follow-up study to the Phase 2B randomized clinical trial in Leigh syndrome, the first randomized trial using a novel redox-modulating agent to treat mitochondrial disease.*
- EPI743 (Enns) 08/20/10–04/14/16 0.12 CM  
Emergency Use Protocol for EPI-743 in Acutely Ill Patients with Inherited Mitochondrial Respiratory Chain Disease Within 90 Days of End-of-life Care  
*This is an emergency treatment protocol for mitochondrial disease patients using a novel redox-modulating agent.*
- SPO117002 (Enns) 04/01/15 – 03/31/16 0.24 CM  
National Institutes of Health  
Lumina Diagnostics, Inc.  
Breath Ammonia Monitoring Device for Children with Urea Cycle Disorders  
*The goal of this project is to develop a novel breath sensor to measure ammonia in patients with urea cycle disorders.*

1R01DK1028201A1 (Peltz) 01/01/15–12/31/17 0.60 CM  
National Institutes of Health \$382,029  
Stem Cell-Based In vivo Models of Human Genetic Liver Diseases  
*The goal of this grant is to generate murine models of genetic liver diseases, including polymerase gamma deficiency and Alagille syndrome, using stem cell techniques to generate “humanized” liver tissue.*

Data and Website (Enns) 07/01/09–06/30/16 0.12 CM  
Genzyme Corporation \$63,885  
Gaucher, Fabry, MPS 1 and other Genetically Based Metabolic Disorders  
*This is a longitudinal registry program for lysosomal storage disorders.*

LAL-CL02 (Enns) 05/31/13–03/31/17 0.60 CM  
Synageva BioPharma Corp. \$406,690  
A Multicenter, Randomized, Placebo-Controlled Study of SBC-102 in Patients with Lysosomal Acid Lipase Deficiency.  
*This is a clinical trial using a novel enzyme replacement therapy to treat LAL deficiency.*

GOS (Enns) 09/16/11–08/31/18 0.12 CM  
Shire Human Genetics Therapies, Inc. \$126,201  
Gaucher Disease Outcome Survey (GOS)  
*This is a longitudinal registry program for Gaucher Disease.*

NIH 14-GG006326 (Enns) 09/01/13–08/31/18 0.01 CM  
North American Mitochondrial Disease Consortium \$580  
*This is a registry program for mitochondrial disease patients.*

Foundation Grant (Enns) 06/01/14–03/31/16 0.12 CM  
Austin Memorial Foundation \$45,873  
A cell-based method for screening mitochondrial disease therapies.  
*This study focuses on the use of spectromicroscopy and tandem mass spectrometry to study fibroblasts obtained from mitochondrial disease patients.*

### C. Employment History:

Academic Appointments: Clinical Instructor, Pediatrics  
University of Southern California  
June, 1994–June, 1995

Clinical Instructor, Pediatrics  
University of California, San Francisco  
July, 1998–present

Assistant Professor of Pediatrics  
Stanford University  
October, 1998–October, 2006

Associate Professor of Pediatrics  
Stanford University  
November, 2006–May, 2015

Professor of Pediatrics  
Stanford University  
June, 2015–present

Administrative  
Appointments:

Director, Biochemical Genetics Program  
Stanford University School of Medicine  
October, 1998–present

Scientific Advisory Board, Genotyping Core  
Stanford University School of Medicine  
May, 2000–June, 2001

Co-Director, UCSF/Stanford Lysosomal Disease Center  
Stanford University Medical Center  
July, 2000–present

Director, Metabolic Special Care Center  
Lucile Salter Packard Children's Hospital  
June, 2001–present

Medical Consultant, Newborn Screening Area Service Center  
Stanford University Medical Center  
June 2003–present

Program Director, Medical Genetics Residency Training Program  
Stanford University School of Medicine  
September, 2007–April, 2013

Associate Program Director, Medical Genetics Residency Training  
Program  
Stanford University School of Medicine  
May, 2013–April, 2014

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