

BIOGRAPHICAL SKETCH

Provide the following information for the key personnel and other significant contributors in the order listed on Form Page 2.
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Barry J. Byrne		POSITION TITLE	
eRA COMMONS USER NAME		Professor of Pediatrics	
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral training.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	YEAR(s)	FIELD OF STUDY
Denison University, Granville, OH	B.S.	1978	Chemistry
University of Illinois, Chicago, IL	M.D./Ph.D.	1984	Microbiology & Immunology
Johns Hopkins University, Baltimore, MD		1991	Resident & Postdoctoral

A. Positions and Honors

1984-1987 Intern and Resident in Pediatrics, Johns Hopkins Hospital, Baltimore, MD
 1987-1991 Fellow in Pediatric Cardiology and Biological Chemistry, Johns Hopkins Univ., Baltimore, MD
 1992-1997 Assistant Professor of Pediatrics, Johns Hopkins University, Baltimore, MD
 1994-1997 Assistant Professor of Pathology, Johns Hopkins University, Baltimore, MD
 1997-1999 Assistant Professor of Pediatrics, Molecular Genetics & Microbiology, University of Florida, Gainesville, FL
 1999-2002 Associate Professor of Pediatrics, Molecular Genetics & Microbiology, Univ. of Florida, Gainesville, FL
 2002- Professor of Pediatrics, Molecular Genetics & Microbiology, Univ. of Florida, Gainesville, FL
 1999- 2002 Co-Director, Powell Gene Therapy Center
 2002 Director, Powell Gene Therapy Center
 2002 Associate Chair for Research, Department of Pediatrics, Univ. of Florida, Gainesville, FL

Other Experience and Professional Memberships

NIH Study Section Member, Skeletal Muscle Exercise Physiology, Chair, 2006-present

NIH ad hoc Study Section Reviewer, 1998-2000.

American Heart Association Reviewer, 1996-present

American Society of Gene Therapy – Member, 1997 - present

Board Certification: Pediatrics and Cardiology

Medical Licensure: State of Florida

Awards and Honors

1983	James Scholar	University of Illinois, Chicago, IL
1984	College of Medicine Research Award	University of Illinois, Chicago, IL
1987	Harriet Lane Home Fellowship Award	Johns Hopkins University, Baltimore, MD
1991-92	Child Health Research Center Award	Johns Hopkins University, Baltimore, MD
1994	Clinician Scientist Award	Johns Hopkins University, Baltimore, MD
1997	Howard Hughes Medical Inst. Award	University of Florida, Gainesville, FL
2003	UF Research Foundation Professor	University of Florida, Gainesville, FL

B. Selected Peer Reviewed Publications (in chronological order):

1. Cahill KS, Toma C, Pittenger MF, Kessler PD, Byrne BJ. Cell therapy in the heart: cell production, transplantation, and applications. Methods Mol Biol., 219:73-81, 2003.
2. Mah C, Sarkar R, Zolotukhin I, Schleissing M, Xiao X, Kazazian HH, Byrne BJ. Dual Vectors Expressing Murine Factor VIII Result in Sustained Correction of Hemophilia A Mice. Human Gene Therapy, Jan 20;14(2):143-52, Jan. 2003.
3. Flotte TR, Brantly ML, Spencer LT, Byrne BJ, Spencer CT, Baker DJ, Humphries M. Phase I trial of intramuscular injection of a recombinant adeno-associated virus alpha 1-antitrypsin (rAAV2-CB-hAAT) gene vector to AAT-deficient adults. Hum Gene Ther.;15(1):93-128, Jan 2004.

PO1 DK58327
NIH/NIDDK

Flotte(PI)

08/01/00 – 07/31/10

“Transduction of Hepatocytes with Recombinant AAV for Correction of Genetic and Metabolic Abnormalities”
Project 3 investigates approach of hepataic gene delivery for systemic correction of GSD II.

Role: Principal Investigator for Proj 3, Byrne

RO1EY012607-03

Grant,M(PI)

12/31/01-12/31/06

“Nitric Oxide in the Pathogenesis of Diabetic Retinopathy” Pathogenesis and Therapy of Complications of Diabetes

Three types of studies will test the hypothesis that high glucose generates chronically increase NO levels that results in endothelial cell dysfunction and impaired blood-retinal integrity responsible for diabetic retinopathy.

Role: Collaborating Investigator, Byrne

Effort: 5%

Muscular Dystrophy Association/NIH
NIH, R01 HL078670 NHLBI

Cahill,KS (PI)

09/20/04 – 08/31/09

“Noninvasive Monitoring and Tracking of Stem Cells”

This study will provide a way to transplant stem cells labeled with paramagnetic molecules that can be tracked non-invasively using MRI.

Role: Co- Principal Investigator, Byrne

Effort: 5%

Bioheart

Byrne(PI)

07/01/04 – 06/31/06

“Adeno-Associated Virus-Modified Human Skeletal Myoblast Therapy for Heart Failure”

The purpose of this study is to demonstrate the potential of Bioheart human skeletal myoblasts to engraft infarcted myocardium as a treatment for heart failure.

Role: Principal Investigator, Byrne

Genzyme

“An Open-Label, Multicenter, Multinational Study of the Safety, Efficacy, Pharmacokinetics, and Pharmacodynamics of Recombinant Human Acid alpha-Glucosidase (rhGAA) Treatment in Patients >6 and <24 months old with Infantile-Onset Glycogen Storage Disease Type II”

Study conducted to evaluate safety and effectiveness of recombinant human acid alpha-glucosidase (rhGAA) as a potential enzyme replacement therapy for Pompe disease in patients >6 and <24 months old.

01/01/03 – 01/27/06

Role: Principal Investigator, Byrne

Genzyme

“A Randomized, Open-label, Multicenter, Multinational, Dose-Ranging Study of the Safety, Efficacy, Pharmacokinetics, and Pharmacodynamics of Recombinant Human Acid alpha-Glucosidase (rhGAA) Treatment in Patients ≤6 months old with Infantile-Onset Pompe Disease.

Study conducted to evaluate safety and effectiveness of recombinant human acid alpha-glucosidase (rhGAA) as a potential enzyme replacement therapy for Pompe disease in patients <6 months old.

01/15/04 – 04/25/06

Role: Principal Investigator, Byrne

5U42RR016586
NIH

Byrne(PI)

09/30/01-08/31/06

“National Gene Vector Laboratory Toxicology Center”

This project provides core services for the preclinical evaluation of AAV toxicology in support of clinical trials.

Role: Principal Investigator

Completed Research Support

R01 DK58236-01

Byrne(PI)

09/15/00 – 7/31/03

NIH/NIDDK

"Glycogen Storage Disease: Hepatic Gene Therapy"

The aims of this proposal are to evaluate the ability of gene transfer in the neonatal period to result in sustained correction of the hepatocellular disease due to absence of glucose-6-phosphatase. Neonatal transduction will be studied in an attempt to rescue newborn animals from severe hypoglycemia at birth.

Role: Principal Investigator

JDFI Center Grant

Atkinson(PI)

10/1/00-8/31/05

Juvenile Diabetes Foundation

"The JDFI Gene Therapy Center for the prevention of diabetes and diabetic complications at the University of Florida and the University of Miami"

Role: Co-PI Core B (Vector Core) (Muzyczka/Byrne) Core B is responsible for supplying AAV vectors to JDFI Center . Members upon request.

Role: Principal Investigator of Pilot: "Induction of Cardiac and Endothelial Allograft Tolerance Using AAV Vectors"

NIH/NHLBI P50 HL59412-01

Muzyczka (PI)

9/1/97 - 8/30/02

"Gene Therapy Using Viral Vectors for Lung and Cardiovascular Disease"

The aims of this proposal are to develop a null mutant of acid alpha-glucosidase and for testing the impact of cardiac gene replacement therapy. AAV vectors expressing acid alpha-glucosidase will be used to achieve long-term correction of the defect in cardiac and skeletal muscle.

Role: Principal Investigator for Proj 1

AHA-50971B

Byrne(PI)

7/1/00 – 6/30/02

AHA - Florida Affiliate: Grant in Aid

"Preclinical Study of Parvovirus Mediated Gene Therapy for Inherited Cardiomyopathy"

The aims of this proposal are to evaluate the preclinical safety of AAV vectors injected into muscle for the correction of inherited cardiomyopathy.

Role: Principal Investigator