Curriculum Vitae

BARRY J. BYRNE

PERSONAL

Address:
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Office:
Powell Center for Rare Disease Research
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EDUCATION

Denison University Granville, Ohio 1974-1978	B.S.	1978	Chemistry
University of Illinois Chicago, Illinois 1978-1984	M.D. Ph.D.	1984 1984	Microbiology & Immunology
The Johns Hopkins Hospital Baltimore, Maryland 1984-1987		Resident in Pediatrics	
Johns Hopkins University Baltimore, Maryland 1987-1991		Postdoctoral Fellow, Biological Chemistry Clinical Fellow, Pediatric Cardiology	

HONORS

Chemistry Departmental Fellow, 1976-1978
Denison University, Granville, Ohio

James Scholar, 1983
University of Illinois, Chicago, Illinois

College of Medicine Research Award, 1984
University of Illinois, Chicago, Illinois

Harriet Lane Home Fellowship Award, 1987
Johns Hopkins University, Baltimore, Maryland

Child Health Research Center Grant Awardee, 1991-1992
Johns Hopkins University, Baltimore, Maryland

Clinician Scientist Award, 1994-1996
Johns Hopkins University, Baltimore, Maryland

Univerisity of Florida, Research Professor Award, 2003-2006
University of Florida, Gainesville, Florida

University of Florida, Faculty Research Prize in Clinical Research, 2007
University of Florida, Gainesville, Florida

PROFESSIONAL EXPERIENCE

2012-present	Earl and Christy Powell University Chair in Genetics Director, Powell Center for Rare Disease Research and Therapy
2006-present	Associate Chair for Research, Department of Pediatrics University of Florida, Gainesville, Florida
2006-2012	Medical Director, Congenital Heart Center University of Florida, Gainesville, Florida
2003-2012	Virginia Root Sutherland Professor of Cardiology Joint Appointments: Dept. of Radiology, College of Medicine; Dept. of Small Animal Science, College of Veterinary Medicine University of Florida, Gainesville, Florida
2002-present	Professor of Pediatrics and Molecular Genetics & Microbiology Director, Powell Gene Therapy Center University of Florida, Gainesville, Florida
1999-2002	Associate Professor of Pediatrics and Molecular Genetics & Microbiology Co-Director, Powell Gene Therapy Center M.D.- Ph.D. Program Director Pediatric Cardiology Fellowship Program Director University of Florida, Gainesville, Florida
1997-1999	Assistant Professor of Pediatrics and Molecular Genetics & Microbiology Gene Therapy Center and Center for Mammalian Genetics Pediatric Cardiology Fellowship Program Director University of Florida, Gainesville, Florida
1994-1999	Assistant Professor of Pathology Johns Hopkins University, Baltimore, Maryland
1992-1997	Assistant Professor of Pediatrics, Pediatric Cardiology Medical Director, Pediatric Cardiac Transplant Program Johns Hopkins University, Baltimore, Maryland

1987-1991	Postdoctoral Fellow with ----(b)(6)----, Department of Biological Chemistry and Clinical Fellow, Division of Pediatric Cardiology, Johns Hopkins University.
1984-1987	Intern and Resident in Pediatrics, The Johns Hopkins Hospital, Baltimore, Maryland
1979-1984	Research Assistant and Teaching Assistant, University of Illinois, Chicago, Illinois
1978-1979	Participant in Great Lakes College Assoc. Independent Study in Science, Biology Division, Oak Ridge National Lab, Oak Ridge, Tennessee
1976-1978	Department of Chemistry Fellow and Teaching Assistant, Denison University, Granville, Ohio

MEMBERSHIP AND PROFESSIONAL ACTIVITIES

Board Certification: Pediarics (1993-2002) and Pediatric Cardiology; 2002-present
Medical Licensure: State of Florida; 1999-present; State of Maryland (inactive)
American Society of Cell and Gene Therapy: Board of Directors, 2012-present
Fellow – American College of Cardiology, 2006 – present
NIH Study Section Chair, Skeletal Muscle Biology, 2006 – 2009.
NIH Study Section Member, Skeletal Muscle Biology, 2001-2008
Society of Regenerative Medicine and Stem Cell Biology- Member, 2000-present
Cardiovascular Diseases in the Young – Member, 1997- present
Fellow - American Academy of Pediatrics; 1993- present
American Pediatric Society / Society for Pediatric Research; 2000-present
International Society for Heart and Lung Transplantation; 1993-present
Co-Chair, Rare Disease Committee, CTSA Consortium-Child Health Oversight Committee; 2009
American Society of Cell and Gene Therapy – Elected to Board of Directors, 2012.

GRANTS and CONTRACTS

ACTIVE

"Gene Therapy Using Viral Vectors for Lung and Cardiovascular Disease"
NIH-P01 HL59412 (PI - Proj 3 and Program PI) $1,267,724 2.40 Celendar
NIH-NHLBI 1/1/2008 – 1/1/2013

"Control of Breathing and Pompe Disease"
R01 HD052682-06 (MPI - Fuller) $320,581 2.40 Calendar
NIH-NICHD 09/01/2012 – 08/31/2017

"Spinal and Brainstem, Respiratory Neurons in Pompe Disease"
R21 NS081431-01 (MPI - Fuller) $150,000 0.60 Calendar
NIH/NINDS 09/01/2012 – 09/01/2014

"Heart and Skeletal Muscle Metabolism, Energetics and Function in Barth Syndrome"
R01 HL107406 (PI-Cade) $211,829 0.60 Calendar
NIH/NHLBI 04/1/2012 – 03/31/2017

"Oral immune modulatory therapy using antigens bioencapsulated in plant cells"
R01 HL106687 (MPI / PI-Herzog) $376,441 0.60 Calendar

NIH/NHLBI	7/01/2011-6/30/2016		

"UFCC for Cardiovascular Cell Therapy Research Network"

U01 HL087366	(Co-PI / PI-Pepine)	$358,850	0.60 Calendar
NIH/NHLBI	1/1/2012-12/31/2019		

"Phase I/II Trial of AAV-GAA in Pompe Disease"

NHLBI, GTRP;	PI	$1,429,722	1.2 Calendar
06/10 – 12/2013			

"Phase I Clincal Study of (b)(4) in Pompe Disease

BioMarin/Zystore,	PI	(b)(4),(b)(6)	0.60 Calendar
12/2010 – 05/2013			

"Planning a multi-center Clinical Trial of PDE5 inhibition for DMD"

U34 AR062893	(PI-Victor)	04/01/2012 – 06/30/2013	0.60 Calendar
NIH/NIAMS		$73,250	

"Magnetic Resonance Imaging and Biomarkers for Muscular Dystrophy"

R01 AR056973-01A1	Co-I	(b)(4),(b)(6)	0.60 Calendar
4/1/2010 – 3/31/2015			

"Failed Regeneration in the Muscular Dystrophies: Inflamation, Fibrosis and Fat"

U54 AR052646 - Senator Paul Wellstone Center			
NINDS/NHLBI 7/1/10 – 6/30/2015	UF Co-PI	$1,239,260	0.60 Calendar

PENDING

"Decoding Immune Responses to Protein and Gene Therapy for Pompe Disease"

R01AI103413-01			
NIH/NIAMS	PI – Byrne	$250,000	1.2 Calendar

RECENT

"Clinical Trials of Gene Therapy for Leber Congenital Amaurosis"

U10 NEI	PI-Byrne	$ 464,494

"National Gene Vector Laboratory Toxicology Center"

NIH, 5U42RR016586,	PI	$4,304,390
09/30/01 – 08/31/10		

"----------(b)(4)---------- Therapy for Heart Failure"

Bioheart, 7/1/04 – 6/31/11	PI- Byrne	PI	(b)(4),(b)(6)

"Pharmacological Chaperone Therapy for Pompe Disease"

Amicus Therapeutics,	PI	(b)(4),(b)(6)
9/1/06 – 5/2/11		

"A Randomized, Open-label, Multicenter, Multinational, Dose-Ranging Study of the Safety, Efficacy, Pharmacokinetics, and Pharmacodynamics of ----------(b)(4)---------- Treatment in Patients ≤ 6 Months Old with Infantile-Onset Pompe Disease
Effective Dates: 1/15/2004 – 4/25/2007, Site PI TDC: (b)(4),(b)(6)

"An Open-Label, Multicenter, Multinational Study of the Safety, Efficacy, Pharmacokinetics, and Pharmacodynamics of ----------(b)(4)---------- Treatment in Patients >6 and <24 months Old with Infantile-Onset Glycogen Storage Disease Type II
Effective Dates:1/ 1/ 2003 – January 27, 2007, PI TDC: (b)(4),(b)(6)

"Preclinical Study of Gene Replacement in Muscular Dystrophy"
Muscular Dystrophy Association, PI- Byrne 1/1/02-9/31/06 (b)(4),(b)(6)

"Glycogen Storage Disease: Hepatic Gene Therapy"
R01 DK58236-01 PI - Byrne/(b)(6) 08/1/2000 - 7/31/2003 (b)(4),(b)(6)

"Preclinical Studies of Enzyme Replacement Therapy for Inherited Cardiomyopathy"
Novazyme Pharmaceuticals, Inc. 9/12/00-4/12/03 (b)(4),(b)(6)

"Cardiac Muscle Regeneration Using Mesenchymal Stem Cells"
NIST-ATP: Osiris Therapeutics / Byrne 12/1/97 - 11/30/2001 (b)(4),(b)(6)

"Preclinical Study of ---------(b)(4)------- Gene Therapy for Inherited Cardiomyopathy"
AHA-50971B 7/1/2000 – 6/30/2002 (b)(4),(b)(6)
AHA - Florida Affiliate Grant in Aid

PATENTS and APPLICATIONS

1. **7,829,078** Methods of treating anemia using recombinant adeno-associated virus virions; July 15, 2008.
2. **7,704,492** Methods for delivering DNA to muscle cells using recombinant adeno-associated virus virions; July 15, 2008.
3. **7,238,674** Methods for delivering DNA to muscle cells using recombinant adeno-associated virus vectors; March 5, 2002.
4. **7,094,604** Production of pseudotyped recombinant AAV virions, March 11, 2004.
5. **6,783,972** Methods for large-scale production of recombinant AAV vectors; Sept. 22, 1999.
6. **6,660,514** Method of preparing recombinant adeno-associated virus compositions; July 21, 2000.
7. **6,610,290** Adeno-associated virus vectors for the treatment of a cardiomyopathy;
8. **6,461,606** Materials and methods for gene therapy; October 1, 2001.
9. **6,335,011** Methods for delivering DNA to muscle cells using recombinant adeno-associated virus virions to treat lysosomal storage disease; Sept. 28, 1999
10. **6,211,163** Methods for delivering DNA to the bloodstream using recombinant adeno-associated virus vectors; May 10, 1999.
11. **6,146,874** Method of preparing recombinant adeno-associated virus compositions; May 10, 1999.
12. **5,962,313** Adeno-associated virus vectors comprising a gene encoding a lyosomal enzyme; Jan. 16, 1996.
13. **5,858,351** Methods for delivering DNA to muscle cells using recombinant adeno-associated virus vectors; Jan. 16, 1996.

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SERVICE ACTIVITES

CTSA – Rare Disease Committee, Co-Chair
CC-CHOC Working group on Core Competency, Chair.
CTSA – KL2 Scholarship Committee, University of Florida
GCRC Scientific Advisory Committee Member
University of Florida Medical School Selection Committee
Associate Editor, *Current Gene Therapy; Cardiology Today*
NIH, Skeletal Muscle Biology Study Section Member and Chair
Director, M.D.- Ph.D. Program

RESEARCH INTERESTS

- Rare disease clinical research.
- Adeno-associated virus mediated gene transfer in the cardiovascular system and striated muscle.
- Cell therapy in cardiovascular disease.
- Gene therapy for muscular dystrophy (Pompe disease and DMD).

INVITED LECTURES

Gene Therapy in Cardiomyopathy and Transplantation
Johns Hopkins University, Pediatrics Grand Rounds, 1995.

AAV Vector Biology and Applications in Therapeutic Protein Production
Johns Hopkins University, 1995.

Genetic Therapies in Cardiomyopathy and Transplantation
Case Western Reserve University, Cleveland, OH, 1996.

Cardiac Gene Therapy
4th Asian Congress of Cardiology, Bangkok, Thailand, 1996.

Gene Therapy using DNA Viral Vectors for Cardiomyopathy
IDP Grand Rounds, University of Florida, 1997

Parvovirus-Mediated Gene Therapy
Society for General Microbiology, Warwick, UK, 1997.

AAV Vectors for Inherited Cardiomyopathy
Inter. Gene Therapy Society Meeting, Heracklion, Crete, 1998.

Parvovirus-mediated Gene Therapy in Cardiovascular Disease
Indiana University, Indianapolis, IN, 1999.

NIH-Italian Conference on Genetic and Pharmacological Approaches in Vascular Biology, Milan, Italy, 1999.

Gene Therapy for the Treatment of Lysosomal Storage Diseases
Southern Society of Pediatric Research, February, 1999.

How to make and use AAV vectors
American Heart Association, 1999.

AAV Vectors for Neuromuscular Disease
UCLA Neuromuscular program and Cedars-Sinai, 2002

AAV Vectors for Treatment of Muscular Dystrophy
MDA Symposium, 2004

Clinical Development of AAV Vectors for Muscular Dystrophy
Monaco Meeting on Muscular Dystrophy, 2004

Sustained Correction of Pompe Disease Using AAV Vectors: Effects of Early Gene Transfer on Cardiac and Pulmonary Function
NHLBI Symposium on Fetal Gene Therapy, 2005

Gene Therapy in Pompe Disease: Pathway to Clinical Studies
Myology, Nantes France, 2005

Clinical Development of AAV Vectors for Muscular Dystrophy
Scripps Institute, 2005

Pompe Disease: Lessons from Preclincial Studies and Future Challenges
World LSD Meeting, 2006

Phase I Clinical Trials of rAAV2 and rAAV1-AAT
International Parvovirus Meeting, 2006

AAV SDF1a-Modified Human Skeletal Myoblast Therapy for Heart Failure
Heart Failure Society, Seattle, 2006.

Cardiac Findings and Outcome at One Year in Children Receiving Myozyme
Steps Forward in Pompe Disease, Berlin, 2006

AAV Vectors for Cardiac Gene Therapy
American Heart Association, 2007

Faculty Participant and Invited Speaker
Children's Hospital of Philadelphia, Cardiology2008

Pompe Disease: Bench to Bedside and Back
Lipman Lecture, OHSU, May 22, 2008

7th Annual Gene Therapy Symposium for Heart Lung and Blood Diseases
Plenary Lecture, 2008

Faculty Participant and Invited Speaker
Children's Hospital of Philadelphia, Cardiology2009

AAV Vectors in Neuromuscular Disease
University of Florida
Center for Exercise Science, 2009

Pharmacological Chaperones in LSD Treatment
World Lysosomal Storage Disease Meeting, San Diego, 2009

3rd Annual Anniversary of Darby Clinical Research Institute, Plenary Talk
Medical University of South Carolina, 2009

Pharmacological Chaperones in LSD Treatment
Americal College of Medical Genetics, Tampa, 2009

Novel Biological Therapies for Inherited Disease in Children: Lessons from Animal Models and Clinical Studies
Asian Society for Pediatric Research, 2010

AAV-Mediated Gene Therapy for Pompe: Beyond the Muscle Disease
Cardiology2010, Plenary Talk, Orlando 2010.

AAV Vectors in Neuromuscular Disease: Bench to Bedside
University of Florida, Muscle Biology Seminar Series, 2009

Contemporary Questions in Congenital Heart Disease
Sick Kids, Toronto, Canada, 2009.

NHLBI Symposium on Cardiovascular Regerative Medicine
Bethesda, MD 2009

Novel Biological Therapies for Inherited Disease in Children: Lessons from Animal Models and Clinical Studies
Asian Society for Pediatric Research, 2010

AAV-Mediated Therapy for Pompe: Beyond the Muscle Disease
Cardiology 2010, 13th Update on Pediatric Cardiovascular Disease

Strategies for Treatment of Inherited Cardiomyopathy: Bench to Bedside
Children's Hospital of Pittsburgh, Grand Rounds, 2010

Faculty and Invited Speaker
Cardiology 2011, 14th Update on Pediatric Cardiovascular Disease

Models of Cardiac Metabolic Dysfunction
American Heart Association Invited Symposium, 2011

Treatment Using AAV-Vectors in Lysosomal Storage Disease
MPS III Symposium, University of Pennsylvania, 2011

Patient Registries in Rare Disease
Health Research Alliance Symposium, 2011

Pompe Disease: Lessons from Preclincial Studies and Future Challenges
World LSD Meeting, 2011

NHLBI Cell Therapy Network
Saudi Heart Association, Rhiad Saudi Arabia, 2011

Models of Cardiac Metabolic Dysfunction
NYU Visiting Professor, 2011

Featured Award in Basic Science Invited Lecture
Children's Hospital of Philadelphia, Caridology-2012, Orlando, 2012.

AAV Vectors in Neuromuscular Disease: Bench to Bedside
University of Florida
Department of Molecular Genetics and Microbiology, 2012

ABSTRACTS

Dhruva, B.R., B.J. Byrne, T. Shenk and K.N. Subramanian. Integration **in vitro** into simian virus 40 of a sequence that resembles a certain family of interspersed repeated sequences. **Cold Spring Harbor 1980 DNA Tumor Virus Meeting**. August, p. 107, 1980.

Byrne, B.J., J. Yamaguchi, D.J. Bergsma and K.N. Subramanian. The SV40 Promoter Regions: a) Localization of the early promoter within the 21-bp repeat; and b) the 72-bp repeat exhibits a pronounced host range in its enhancement effect. **Cold Spring Harbor 1982 DNA Tumor Virus Meeting**. August, p. 13, 1982.

Hartzell, S.W., B.J. Byrne, and K.N. Subramanian. The definition of the boundaries of the SV40 late promoter. **ICRF DNA Tumor Virus Meeting**, 1983.

Byrne, B.J., Kaczorowski, Y.H., Larson-Kelly, N., and S.W. Craig. Vinculin and Meta-vinculin are alternatively spliced products of a single gene. **Society for Pediatric Research**, 1990.

Byrne, B.J., Nicholson, K.L., and S.W. Craig. Meta-vinculin is expressed in myocardium of avian embryos with the onset of cardiac activity. **ASCB**, 1992.

Recchia, F.A., Byrne, B.J., Kass, D.A. Vasorelaxant Effect of Physiologic Arterial Pulsatility in Isolated Vessels. **Circulation** 90:4 357a, 1994.

Kourtis, A.P., Deyesu, E.E., Hruban R.H., Byrne B.J. Cardiac gene therapy with adeno-associated virus as a means of achieving graft-specific immunosuppression. **Mod Pathol** 8:33A, 1995.

Pauly, D., D. Johns, E. Deyesu, M. Lowery, J. Lawrence, T. Flotte, B. Byrne, P. Kessler. Acid - glucosidase deficiency: A model system for gene transfer into cardiac muscle. **J. Cell. Biochem. Suppl**. 21a: 370; 1995.

Kessler, P.D., Matelis, L.A., Wei, S. -K., Silverman, H.S., Flotte, T. R., Kurtzman, G.J., and B.J. Byrne. Sodium Butyrate Greatly Enhances the Efficiency of Viral Transduction in Adult Ventricular Cardiomyocytes by Adeno-associated Viral Vectors. **Circulation**, (92): I-296; 1995.

Podsakoff G, Carper E, Colosi P, McQuiston S, Chen X, Cao S, Kessler P, Byrne B, Kurtzman. Long-term in vivo gene expression in muscle using AAV vectors. **Blood** 86 (10) Suppl:1004a; 1995 *Presented American Society of Hematology, 1996.*

Kessler, P.D., Byrne, B.J., Chen, X., Matelis, LA, McQuinston, S, Kurtzman, G, Podsakoff, GM. Gene delivery to skeletal muscle results in the sustained expression and systemic delivery of therapeutic proteins. **J. Invest. Med**. 44:279a; 1996.

Podsakoff GM, Nakai H, McQuiston SA, Kessler PD, Byrne BJ, Couto LB, Kurtzman GJ. AAV vector-mediated gene delivery to skeletal muscle in vivo results in sustained levels of systemic erythropoietin. **Blood** 88 (10) Suppl 1: 509-II; 1996.

Byrne, B.J., Brust, S., Chen, X.X., Kessler, P.D., Flotte, T.R. and W. B. Guggino. Recombinant adeno-associated virus in embryonic gene therapy. Weinstein Cardiovascular Development, 1996.

Byrne BJ, Zhang G, Pauly DF, Matelis LA, Chen X, Podsakoff GM, Colosi P, Kurtzman GJ, Guggino WB, & Kessler PD AAV-mediated gene therapy for an inherited form of hypertrophic cardiomyopathy. **Circulation** 94: I-119; 1996.

Chen X, Kessler PD, Podsakoff GM, Kurtzman GJ, Byrne BJ. Long-term gene expression of adeno-associated virus-mediated gene transfer in mouse skeletal muscle in vivo. **Circulation** 94: I-43; 1996.

Conway, J., Byrne, B. and G. Hayward. GFP as a marker of transduction in an HSV-1 amplicon gene transfer system. 21st International HSV Workshop, 1996.

Conway, J., Hayward, G., and B.J. Byrne. Recombinant adeno-associated virus replication and packaging is supported by an HSV amplicon expressing *rep* and *cap*. **Keystone Symposia**, 1997.

Byrne BJ, Pauly DF, Zhang G, Chen X, Guggino WB, Kurtzman GJ, Kessler PD. Gene replacement in acid maltase deficiency using DNA Viral Vectors. **Ped Res**. 41 (4) Part 2: 19A; 1997

Pauly DP, Byrne BJ, Bayes HS, Kessler PD. Correction of the enzyme deficiency in an hereditary cardiomyopathy through passive transfer of recombinant enzyme. **Circulation** 96: Suppl. I: I-18; 1997.

Bayes, H, Pauly, D., Johns, D., Lawrence, J., Kessler, P., and B.J. Byrne. Therapeutic Strategy for Lysosomal Storage Disease Based on DNA Viral Vectors. Amer. Soc. Gene Therapy 1st Annual Meeting, May 28-31st, 74a, 1998.

Song, S., Morgan, M., Ellis, T., Poirier, A., Chesnut, K., Wang, J., Brantly, M., Atkinson, M., Byrne, B.J., and TR Flotte. Sustained Secretion of Human Alpha-1 Antitypic from Murine Muscle Transduced with Adeno-associated Virus Vectors., **Amer. Soc. Gene Therapy**, 1998.

Conway, J., C. M. J. Ap Rhys, I. Zolotukhin, G.S. Hayward and B.J. Byrne, High Titer Recombinant Adeno-Associated Virus Production Utilizing A Replication Defective Recombinant Herpes Virus Expressing Aav-2 Rep And Cap And The Hsv-1 Helper Functions. **Cold Spring Harbor Symposium**, 1998.

Byrne, B.J., Raben, N., Plotz, P., Fraites, T., and P.D. Kessler., Reconstitution of Acid α–Glucosidase Activity in a Mouse Model of Cardiomyopathy, Pompe Disease. Platform Presentation, American Heart Association, **Circulation** 98: Suppl. I: I-737, 1998.

Toma C, Byrne BJ, Golub J, Gruber PJ, Murry CE Pittenger MF, Kessler PD. Efficient myogenic conversion of human mesenchymal stem cells with forced expression of MyoD. **Circulation** 100: Suppl. I: 1-164, 1999.

Pauly D, Fraites T, Schlessing, Cloutier D, Kessler PD, Byrne BJ. Correction and Transfer of Enzyme Activity Using Adenovirus encoding Human and Murine Acid a-glucosidase. Amer. Soc. Gene Therapy 2nd Annual Meeting, p 178a, June 9-13, 1999.

Toma C, Pittenger M, Byrne BJ, Kessler PD. Adult Human Mesenchymal Cells Differentiate to a Striated Muscle Phenotype following Arterial Delivery to the Murine Heart. **Circulation** 102: Suppl. II: II-683, 2000.

Fraites TJ, Schlessing MR, Owens KM, Cloutier DA, Zolotukhin I, Raben N, Plotz PH, Kessler PD, Byrne BJ. Recombinant adeno-associated virus mediated correction of murine acid a-glucosidase deficiency. **Molecular Ther** 1: Part 2 of 2; S254, 2000.

Cresawn,KO, Porvasnik,SL, Raben,R, Plotz, PR and BJ Byrne. Inhibition of Humoral Immunity in Liver-Targeted Gene Therapy for Glycogen Storage Disease Type II. American Society of Gene Therapy 5th Annual Meeting, June 5-9, 2002. **Molecular Ther.,** Vol 5, (5) Part 2 of 2; S342, May 2002.

Schleissing, MR, Porvasnik, SL and BJ Byrne. Delivery of Recombinant Adeno-Associated Virus Encoding Human Acid Alpha-Glucosidase through *Intra-Uterine* Injection in a Murine Model of Pompe Disease. American Society of Gene Therapy 5th Annual Meeting, June 5-9, 2002. **Molecular Ther.,** Vol 5, (5) Part 2 of 2; S362, May 2002.

Mah, Cathryn; Cresawn, Kerry O; DeRuisseau, Lara R; Fuller, David; Byrne, Barry J: Correction of Respiratory Function by Recombinant AAV1 Mediated Gene Therapy ina Murine Model of glycogen Storage Disease Type II. Poster Session. Gene Therapy Approaches to Pulmonary Disease. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO.

Pacak, Christina A; Mah, Cathryn; Gaidosh, Gabriel; Lewis, Melissa; Torres, Raquel; Campbell, Kevin; Walter, Glenn A; Byrne, Barry J: Development of AAV-Mediated Gene Therapy for Murine Models of Genetic Diseases Affecting the Heart. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO

Kohlbrenner, Erik; Aslanidi, George; Nash, Kevin; Shklayev, Stanislav; Campbell-Thompson, Martha; Byrne, Barry J; Snyder, Richard O; Muzyczka, Nicholas; Warrington, Kenneth H; and Zolotukhin; Sergei: Successful Production of Pseudotyped Mosaic rAAV Vectors Using a Modified Baculovirus Expression System. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO

Young-Kook, Choi; Zolotukhin, Irene; Byrne, Barry J; and Song, Sihong: Effect of DNA-PKcs on AAV Replication. Poster Session: AAV Vectors: Vector Biology. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO

DeRuisseau, Lara R; Mah, Cathryn S; Farites, Thomas J; Fuller, David D; and Byrne, Barry J: Recombinant AAV1-Mediated Treatment of CNS Glycogen Accumulation in Mouse Model of Glycogen Storage Disease Type II. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO

Cresawn, Kerry O; Mah, Cathryn S; Fraites, Thomas J; Lewis, Melissa A; zolotukhin, Irene; and Byrne, Barry J: Sustained Correction of Glycogen Storage Disease Type II by rAAV1 Vector-Mediated Gene Therapy. Poster Session: Inborn Errors of Metabolism: Liver and Pancreatic Diseases. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO

Cresawn, Kerry O; Mah, Cathryn S; Zolotukhin, Irene; Lewis, Melissa A; and Byrne, Barry J: Comparison of rAAV Serotype, Promotor, and Treatment Age for the Correction of Glycogen Storage Disease Type II. Poster Session: Inborn Errors of Metabolism: Liver and Pancreatic Diseases. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO

Brantly, Mark L; Humphries Margaret; Song, Sihong; Conlon, Thomas; Poirier, Amy; Byrne, Barry J; Snyder, Richard, and Flotte, Terence R: Phase I Clinical Trial of Recombinant Adeno-Associated Virus (rAAV)-Alpha-1 Antitrypsin Vectors. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO

Porvasnik, Stacy L; Onal, Tankut; Sakai, Yoshi; Torres, Raquel; Walter, Glenn A; Byrne, Barry J; and Spencer, Carolyn T: Gene Therapy in a Rat Model of Pulmonary Hypertension. American Society of Gene Therapy 8th Annual Meeting, June 1-5, 2005, St. Louis, MO

Simon, Greg; Wasserfall, Clive; Byrne, Barry; and Atkinson, Mark: Prevention of Type 1 Diabetes in the NOD Mouse. The 8th Meeting of the Immunology of Diabetes Society (IDS-8). October 6-9, 2005.

Pacak, C.A., C. Mah, B. Thattaliyath, M.A. Lewis, S. Germain, and B.J. Byrne (2006). The AAV9 Capsid Preferentially Transduces Cardiac Tissue and demonstrates unique behavior In Vivo. XIth Parvovirus Workshop. Les Diablerets, Switzerland.

Pacak, C., C. Mah, B. Thattaliyath, M. Lewis, S. Germain, G. Walter, and B.J. Byrne (2006). AAV9-Mediated Gene Delivery Corrects the Cardiac Phenotype in a Mouse Model of Pompe Disease. 5th Annual Gene Therapy Symposium for Heart, Lung, and Blood Diseases. Sonoma, California.

Mah, C., C.A. Pacak, K.O. Cresawn, L.R. DeRuisseau, S. Gremain, M.A. Lewis, D.D. Fuller, and B.J. Byrne (2006). Physiological Correction of Pompe Disease using Adeno-Associated Serotype 1 Vectors. XIth Parvovirus Workshop. Les Diablerets, Switzerland. Oral Presentation.

Pacak, C.A., C. Mah, K.O. Cresawn, M.A. Lewis, S. Germain, and B.J. Byrne (2006). rAAV2/9 Mediated Gene Delivery of Acid a-Glucosidase Corrects the Cardiac Phenotype in a Mouse Model of Pompe Disease. The American Society of Gene Therapy 9th Annual Meeting, Baltimore, Maryland.

Mah, C., L.R. DeRuisseau, C.A. Pacak, M.A. Lewis, D.D. Fuller, and B.J. Byrne (2006). Correction of Ventilation in Glycogen Storage Disease Type II Mice and Gel-Mediated Delivery of Adeno-Associated Virus Serotype 1 Vectors. The American Society of Gene Therapy 9th Annual Meeting, Baltimore, Maryland.

DeRuisseau, L.R., C. Mah, D.D. Fuller, and B.J. Byrne (2006). Neural Deficits Contribute to respiratory Insufficiency in Pompe Disease: A Therapeutic Approach with AAV1. The American Society of Gene Therapy 9th Annual Meeting, Baltimore, Maryland.

Mah, C., C.A. Pacak, K.O. Cresawn, L.R. DeRuisseau, S. Germain, M.A. Lewis, D.D. Fuller, and B.J. Byrne (2006). Physiological Correction of Glycogen Storage Disease Type II using Adeno-Associated Virus Serotype 1 Vectors. The American Society of Gene Therapy 9th Annual Meeting, Baltimore, Maryland. Oral Presentation.

DeRuisseau, L.R., C. Mah, B.J. Byrne, and D.D. Fuller (2006). Neural Control of Breathing: Disease and Plasticity (2006). Experimental Biology Meeting Abstracts. The FASEB Journal, 20, Abstract #750.

CA Pacak, G Gaidosh, S Germain, M Lewis, GA. Walter, BJ Byrne (2006). Muscle Specific Delivery of the Alpha Sarcoglycan Gene Provides Functional and Morphological Correction of Limb Girdle Muscular Dystrophy Type 2D, Pediatric Science Days, 2006.

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