



The 6th Congress of Asian Society for Pediatric Research
& 51st Annual Meeting of Taiwan Pediatric Association

April 15-18, 2010 Taipei, Taiwan



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PROFESSIONAL EXPERIENCES :

- ◆ 2006-present Associate Chair for Research, Department of Pediatrics
Medical Director, Congenital Heart Center
University of Florida, Gainesville, Florida
 - ◆ 2003-present Virginia Root Sutherland Professor of Cardiology
University of Florida, Gainesville, Florida
 - ◆ 2002-present Professor of Pediatrics and Molecular Genetics & Microbiology
Associate Chair for Research
Director, Powell Gene Therapy Center
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EDUCATIONAL BACKGROUND :

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

- ◆ Adeno-associated virus mediated gene transfer in the cardiovascular system and striated muscle.
 - ◆ Cell therapy in cardiovascular disease.
 - ◆ Gene therapy for glycogen storage disease II (Pompe disease).
 - ◆ Mechanisms of allograft tolerance in cardiac transplantation.
 - ◆ Cardiac development.
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PUBLISHED WORKS : (Selected list)

- Nicolino M, Byrne B, Wraith JE, Leslie N, Mandel H, Freyer DR, Arnold GL, Pivnick EK, Ottinger CJ, Robinson PH, Loo JC, Smitka M, Jardine P, Tatò L, Chabrol B, McCandless S, Kimura S, Mehta L, Bali D, Skrinar A, Morgan C, Rangachari L, Corzo D, Kishnani PS. Clinical outcomes after long-term treatment with alglucosidase alfa in infants and children with advanced Pompe disease. *Genet Med.* 2009 Mar;11(3):210-
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- Mitchell M, Nam HJ, Carter A, McCall A, Rence C, Bennett A, Gurda B, McKenna R, Porter M, Sakai Y, Byrne BJ, Muzyczka N, Aslanidi G, Zolotukhin S, Agbandje-McKenna M. Production, purification and preliminary X-ray crystallographic studies of adeno-associated virus serotype 9. *Acta Crystallogr Sect F Struct Biol Cryst Commun.* 2009 Jul 1. Epub 2009 Jun 27.
- Clement N, Knop Dr, Byrne BJ. Large-Scale Adeno-Associated Viral Vector Production Using a Herpesvirus-Based System Enables Manufacturing for Clinical Studies. *Hum Gene Ther.* 2009 Jul 1. [Epub ahead of print].
- Jacobs JP, Quintessenza JA, Burke RP, Bleiweis MS, Byrne Bj, Ceithaml EL, DeCampli WM, Giroud JM, Perryman RA, Rosenkranz, ER, Wolff G, Posner V, Sterverson S, Blanchard WB, Schiebler GL. Analysis of regional congenital cardiac surgical outcomes in Florida using The Society of Thoracic Surgeons Congenital Heart Surgery Database. *Cardiol Young,* 2009 July 6:1-10. [Epub ahead of print]
- Mendell JR, Louise R. Rodino-Klapac, Xiomara Rosales-Quintero, Janaiah Kota, Brian D. Coley, Gloria Galloway, Josepha M. Craenen, Sarah Lewis, Vinod Malik, Christopher Shilling, Barry J. Byrne, Thomas Conlon, Katherine J Campbell, William G. Bremer, Laurence Viollet, Christopher M. Walker, Zarife Sahenk, K. Reed Clark. LGMD 2D gene therapy restores alpha-sarcoglycan and associated proteins. *Ann. Neuro.* (2009), ePrint.
- Mah, C, Lewis, MA, Conlon, TA, Cloutier, DA, Fuller, DD, DeRuisseau, LR and Byrne, BJ. (2008). Gel-Mediated Delivery of Adeno-Associated Virus Serotype 1 Vector to the Diaphragm Corrects Ventilatory Function in Pompe Mice with Progressive Disease. *Mol. Ther.* (2009), in revision.
- Brantly M, Chulay J, Wang L, Christian Mueller, Humphries M, Spencer T, Rouhani F, Conlon T, Calcedo R, Betts M, Spencer C, Byrne BJ, Wilson J, Flotte TR. Sustained Expression of Alpha-1 Antitrypsin Despite Effector T Cell Responses in a Phase 1 Clinical Trial of Intramuscular rAAV1-AAT, *PNAS (USA)* 2009, submitted.
- Cideciyan AV, Aleman TS, Boye SL, Schwartz SB, Kaushal S, Roman AJ, Pang JJ, Sumaroka A, Windsor EA, Wilson JM, Flotte TR, Fishman GA, Heon E, Stone EM, Byrne BJ, Hauswirth WW, Jacobson SG. Emergence of New Useful Vision One Year after Retinal Gene Therapy for Leber Congenital Amaurosis. *NEJM* (2009), submitted.
- Herzog R, Martino A, Nayak S, Hoffman B, Cooper M, Liao G, Markusic D, Byrne BJ, Terhorst C. Tolerance Induction to Cytoplasmic β -Galactosidase by Hepatic AAV Gene Transfer – Implications for Antigen Presentation and Immunotoxicity. 09-PONE-RA-10491 (2009), submitted.