

# SIHONG SONG

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## EDUCATION

1982        **B.S. in Animal Science**, Jilin Agricultural University, Changchun, P.R. China  
1989        **M.S. in Animal Science**, Jilin Agricultural University, Changchun, P.R. China  
1996        **Ph.D. in Animal Molecular & Cell Biology**, University of Florida, Gainesville, FL  
1996-1999 **Post Doctoral Associate in Gene Therapy**, University of Florida, Gainesville, FL

## POSITIONS

1982-1986    **Teaching and Research Assistant (Faculty)**  
Department of Animal Science, Jilin Agricultural University, China.

1987-1991    **Lecturer (Faculty)**  
Jilin Agriculture University, China

1992-1996    **Graduate Assistant**  
Department of Dairy and Poultry Sciences, University of Florida.

1996-1999    **Post Doctoral Associate**  
Department of Molecular Genetics and Microbiology, University of Florida

1999-2001.7 **Research Assistant Professor**  
Department of Pediatrics, Powell Gene Therapy Center, University of Florida

2001.8-2006.6 **Assistant Professor**  
Department of Pharmaceutics, University of Florida College of Pharmacy

2006.7-present **Associate Professor**  
Department of Pharmaceutics, University of Florida College of Pharmacy

## HONORS

- 1999 Young Investigator Fellowship Awards of the Alpha One Foundation.
- 2005 Junior Faculty Research Award of Sigma Xi, The Scientific Research Society.

- 2008 Global Arthritis Research Network (GARN) award
- 2009-2011 University of Florida Research Foundation (UFRF) Professorship Award.

## MEMBERSHIPS

- Member of the American Society of Gene & Cell Therapy (ASGCT)
- Member of the American Diabetes Association (ADA)
- Member of Sigma Xi, The Scientific Research Society

## PUBLICATIONS

Wang, C., Song, S., Bailey, L.B., Gregory, J. F. *Relationship between urinary excretion of p-aminobezoylglutamate and folate status of growing rats.* **Nutrition Research** 1994, 14: 875-884.

Song, S., Lee, C.Y., Green, M.L., Chung, C.S., Simmen R.C.M., Simmen, F.A. *The unique endometrial expression and genomic organization of the porcine IGFBP-2 Gene.* **Molecular and Cellular Endocrinology** 1996, 120:193-202.

Badinga, L., Song, S., Simmen, R.C.M., Simmen, F.A. *A distal regulatory region of the insulin-like growth factor binding protein-2 (IGFBP-2) gene interacts with the basic helix-loop-helix transcription factor, AP-4.* **Endocrine** 1998, 8(3):281-289.

Simmen, F.A., Badinga, L., Green, M.L., Kwak, I., Song, S. and Simmen, R.C.M. *The Porcine IGF System: At the Interface of Nutrition, Growth and Reproduction.* **Journal of Nutrition** 1998, 128:315s-320s.

Song, S., Morgan, M., Ellis, T., Poirier, A., Chesnut, K., Wang, J., Brantly, M., Byrne, B.J., Atkinson, M., Muzyczka, N., Flotte, T.R. *Sustained secretion of human alpha-1-antitrypsin from murine muscle transduced with adeno-associated virus vectors.* **Proc. Natl. Acad. Sci. USA.** 1998, 95(24):14384-14388.

Badinga, L., Song, S., Simmen, R.C.M., Clarke, J.B., Clemons, D.R., Simmen, F.A. *Complex mediation of uterine endometrial epithelial cell growth by insulin-like growth factor-II (IGF-II) and IGF-binding protein-2.* **Journal of Molecular Endocrinology** 1999, 23:277-285.

Xu, L., Daly, T., Gao, C., Byrne, B.J., Flotte, T.R., Song, S., Sands, M.S., Ponder, K.P. *The CMV- $\beta$ -actin promoter directed higher expression from an AAV vector in the liver than the CMV or EF1 $\alpha$  promoter and results in therapeutic levels of human factor X in mice.* **Human Gene Therapy** 2001, 12:563-573.

Flotte, T.R., Agarwal, A., Wang, J., Song, S., Fenjves, E.S., Inverardi, L., Chesnut, K., Fione, S., Loiler, S., Wasserfall, C., Kapturczak, M., Ellis, T., Nick, H., Atkinson, M. *Efficient ex vivo transduction of pancreatic islet cell with recombinant adeno-associated virus vectors.* **Diabetes** 2001, 50:515-520.

- Song, S.**, Embury, J. Laipis, P.J., Berns, K.I., Crawford J.M., Flotte, T.R. *Stable therapeutic serum levels of human alpha-1 antitrypsin (AAT) after portal vein injection of recombinant adeno-associated virus (rAAV) vectors.* **Gene Therapy**, 2001,8:1299-1306.
- Song, S.**, Laipis, P.J., Berns, K.I., Flotte, T.R. *Effect of DNA-PKcs on the molecular fate of the rAAV2 genome in skeletal muscle.* **Proc. Natl. Acad. Sci. USA.** 2001, 98(7):4084-4088.
- Goudy, K., **Song, S.**, Wasserfall, C., Zhang, Y.C., Kapturczak, M., Muir, A., Scott-Jorgensen, M., Campbell-Thompson, M., Crawford, J.M., Ellis, T.M., Flotte, T.R., and Atkinson, M.A. *Adeno-associated virus vector-mediated IL-10 gene delivery prevents type 1 diabetes in NOD mice.* **Proc. Natl. Acad. Sci. USA.** 2001, 98(24):13913-13918.
- Mah, C., Fraites, T.J., Zolotukhin I., **Song, S.**, Flotte, T.R., Batich, C., Byrne B.J. *Improved method of recombinant AAV2 delivery for systemic targeted gene therapy.* **Molecular Therapy** 2002, 6(1):106-112.
- Song, S.**, Scott-Jorgensen, M., Wang, J., Poirier, A., Crawford, J., Campbell-Thompson, M., Flotte, T.R., *Recombinant adeno-associated virus 2-alpha 1-antitrypsin (rAAV-AAT) vectors in a non-human primate model: safety and immunologic aspects.* **Molecular Therapy** 2002, 6(3):329-335
- Goudy, K., Burkhardt, B., Wasserfall, C., **Song, S.**, Campbell-Thompson, M., Brusko, T., Powers, M., Clare-Salzler, M., Sobel, E., Ellis, T., Flotte, T.R., and Atkinson, M.A. *Systemic Over Expression of IL-10 Induces CD4+ CD25+ Cell Populations In Vivo and Ameliorates Type 1 Diabetes in nonobese diabetic Mice in a Dose-Dependent Fashion.* **Journal of Immunology** 2003, 171:2270-2278.
- Loiler, S.A., Conlon, C.J., **Song, S.**, Tang, Q., Warrington, K.H., Agarwal, A., Kapturczak, M., Li, C., Ricordi, C., Atkinson, M.A., Muzyczka, N., Flotte, T.R. *Targeting Recombinant Adeno-Associated Virus Vectors to Enhance Gene Transfer.* **Gene Therapy** 2003, 10:1551-1558.
- Zhang, Y.C., Powers, M., Wasserfall, C., Brusko, T., **Song, S.**, Flotte, T.R., Snyder, R.O., Potter, M., Scott-Jorgensen, M., Campbell-Thompson, M., Crawford, J.M., Nick, H.S., Agarwal, A., Ellis, T.M., and Atkinson, M.A. *Immunity to adeno-associated virus serotype 2 delivered transgenes imparted by genetic predisposition to autoimmunity.* **Gene Therapy** 2004, 11:233-240
- Song, S.\***, Lu, Y.\*\*, Choi, Y.\*\*, Han, Y-K., Tang, Q., Zhao, G.\*\*, Berns, KI and Flotte, T.R. *DNA dependent Protein Kinase (DNA-PK) inhibits adeno-associated virus (AAV) integration.* **Proc. Natl. Acad. Sci. USA.** 2004, 101(7): 2112-2116.
- Song, S.\***, K Goudy, M Campbell-Thompson, C Wasserfall, M Scott-Jorgensen, J Wang, Q Tang, JM Crawford, TM Ellis, MA Atkinson, and TR Flotte. *Recombinant adeno-associated virus (rAAV2) mediated alpha-1 antitrypsin gene therapy prevents type 1 diabetes in NOD mice.* **Gene Therapy** 2004, 11: 181-186.
- Wei, F-S., **Song, S.**, Zhao, G.\*\*, Lu, Y.\*\*, Li, C., Wei, J-F. And Flotte, T.R. *Stable transduction of murine embryonic stem (ES) cells with recombinant adeno-associated virus serotype 2 (AAV2) vectors.* **Preclinica** 2004, 2(4).

Poirier, A., Campbell-Thompson, M, Tang, Q., Scott-Jorgensen, M., Combee, L, Loiler, S., Crawford, J., **Song, S.**, Flotte, T.R. *Toxicology and Biodistribution Studies of a Recombinant Adeno-associated virus 2 (rAAV2)-Alpha-1 Antitrypsin (AAT) Vector*. **Preclinica** 2004, 2(1): 43-51.

**Song, S.\***, Witek, R. P., Lu, Y\*\*., Choi, Y-K.\*\*., Zheng, D., Jorgensen, M., Li, C., Flotte, T. R., Petersen, B. E. *Ex vivo transduced liver stem cells as a platform for gene therapy*. **Hepatology** 2004, 40:918-924.

Virella-Lowell, I., Zusman, B., Forst, K., Loiler, S., Conlon, T., **Song, S.**, Chesnut, K. A., Ferkol, T., Flotte, T. R. *Enhancing rAAV vector expression in the lung*. **J Gene Med** 2005, 7:842-850

Conlon, T., Cossette, T., Erger, K., Choi, Y-K.\*\*., Clarke, T., Scott-Jorgensen, M., **Song, S.**, Campbell-Thomson, M., Crawford, J. and Flotte T. *Efficient Hepatic Delivery and expression from a recombinant adeno-associated virus 8 (rAAV8) pseudotyped alpha-1 antitrypsin vector*. **Molecular Therapy** 2005, 12 (5):867-875

Burkhardt, B.R., Parker, M.J., Zhang, Y.C., **Song, S.**, Wasserfall, C.H., Atkinson, M.A. *Glucose transporter-2 (GLUT2) promoter mediated transgenic insulin production reduces hyperglycemia in diabetic mice*. **FEBS Letters** 2005, 579:5759-5764

Kwak, I., **Song, S.**, Blum, J.L. Simmen, R.C.M. and Simmen, F.A. *Enhancer- and Silencer-like Sequences that Mediate Insulin-Like Growth Factor-Binding Protein-2 Gene Expression in Uterine Cells of Pregnancy*. **DNA and Cell Biology** 2005, 25(1):6-18.

Petrache, I., Fijalkowska, I., Zhen, L., Medler1, T.R., Brown, E., Cruz, P., Choe, K-H., Taraseviciene-Stewart, L., Scerbavicius, R., Shapiro, L., Zhang, B.\*\*., **Song, S.**, Hicklin, D., Voelkel, N.F., Flotte, T., and Tudert, R.M. *A novel anti-apoptotic role for alpha 1 antitrypsin in the prevention of emphysema*. **Am J Respir Crit Care Med** 2006, 173:1222-1228.

Lu, Y.\*\*., Choi, Y-K.\*\*., Campbell-Thompson, M., Li, C., Tang, Q., Crawford, J., Flotte, T. R. and **Song, S.\*** *Therapeutic level of functional human alpha 1 antitrypsin (hAAT) secreted from murine muscle transduced by adeno-associated virus (rAAV1) vector*. **Journal of Gene Medicine**, 2006, 8:730-735.

Brantly, M.L., Spencer, L.T., Humphries, M., Conlon, T.J., Spencer, C.T., Poirier, A., **Song, S.**, Berns, K., Muzyczks, N Snyder R.O., Byrne, B.J., Flotte, T.R. *Phase I Trial of Intramuscular Injection of a Recombinant Adeno-associated Virus Serotype 2 (rAAV2) Alpha-1 Antitrypsin (AAT) Vector in AAT-deficient Adults*. **Human Gene Therapy** 2006, 17(12):1177-1186.

Lu, Y.\*\*., Tang, M.\*\*., Wasserfall, C., Kou, Z., Campbell-Thompson, M., Gardemann, T.\*\*., Crawford, J., Atkinson, M., and **Song, S.\***  *$\alpha 1$ -antitrypsin gene therapy modulates cellular immunity and efficiently prevents type 1 diabetes in nonobese diabetic mice*. **Human Gene Therapy** 2006, 17 (6):625-634.

Zhang, B.\*\*., Lu, Y.\*\*., Campbell-Thompson, M., Spencer, T., Wasserfall, C., Atkinson, M., **Song, S.\*** *Alpha 1 antitrypsin protects  $\beta$ -cells from apoptosis*. **Diabetes** 2007 56:1316-1323.

Lu, Y.\*\*, Parker, M., Pileggi, A., Zhang, B.\*\*, Choi, Y-K.\*\*, Molano, R.D., Wasserfall, C., Ricordi, C., Inverardi, L., Brantly, B., Schatz, D., Atkinson, M and **Song, S.\*** *Human Alpha 1-Antitrypsin Therapy Induces Fatal Anaphylaxis in NOD Mice.* **Clinical & Experimental Immunology** 2008, 154: 15-21.

Molano, R.D., Pileggi, A., **Song, S.**, Zahr, E., SanJose, S., Molina, J., Fort, A., Wasserfall, C., Ricordi, C., Atkinson, M.A., and Inverardi, L. *Prolonged Islet Allograft Survival by Alpha-1 Antitrypsin: The Role of Humoral Immunity,* **Transplantation Proceedings** 2008, 40:455– 456.

Pileggi, A., Molano, R.D., Song, S., Zahr, E., SanJose, S., Villate, S., Wasserfall, C., Ricordi, C., Atkinson, M.A., and Inverardi, L. *Alpha-1 Antitrypsin Treatment of Spontaneously Diabetic Nonobese Diabetic Mice Receiving Islet Allografts,* **Transplantation Proceedings** 2008, 40:457–458.

Lu, Y.\*\*, **Song, S.\*** Distinct immune responses to transgene products from rAAV1 and rAAV8 vectors. **Proc. Natl. Acad. Sci. USA.** 2009, 106(40): 17158-17162.

Parker, M., Xue, S., Alexander, J., Wasserfall, C., Campbell-Thompson, M., Battaglia, M., Gregori, S., Mathews, C., **Song, S.**, Troutt, M., Eisenbeis, S., Williams, J., Schatz, D., Haller, M., Atkinson, M. *Immune depletion with cellular mobilization imparts immunoregulation and reverses autoimmune diabetes in NOD mice.* **Diabetes** 2009, 58:2277-2284.

Ma, H. \*\*, Lu, Y.\*\*, Li, H.\*\*, Campbell-Thompson, M., Parker, M., Wasserfall, C., Haller, M., Brantly, B., Schatz, D., Atkinson, M., and **Song, S.\*** *Intradermal  $\alpha 1$ -antitrypsin therapy avoids fatal anaphylaxis, prevents type 1 diabetes and reverses hyperglycaemia in the NOD mouse model of the disease.* **Diabetologia** 2010, 53:2198–2204.

Li, H.\*\*, Lu, Y.\*\*, Witek, R.P.\*\*, Chang, L-J., Campbell-Thompson, M., Jorgensen, M., Petersen, B., and **Song, S.\*** *Ex vivo Transduction and Transplantation of Bone Marrow Cells for Liver Gene Delivery of Alpha 1 Antitrypsin.* **Molecular Therapy** 2010, **18(8):1553-1558.**

Grimstein, C.\*\*, Choi, Y-K.\*\*, Satoh, M., Brantly, M., Campbell-Thompson, M., and **Song, S.\*** *Doxycycline regulated alpha-1 antitrypsin (AAT) gene therapy prevents collagen induced arthritis in mice.* **Journal of Gene Medicine** 2010, 12:35-44.

Choi, Y-K.\*\*, Nash, K., Byrne, B., Muzyczka, N. and **Song, S.\*** *The effect of DNA-dependent protein kinase on AAV replication,* **PLoS ONE** 2010, 5(12):e15073.

Pinto, E., Zhang, B.\*\*, **Song, S.**, Bodor, N., Buchwald, P., Hochhaus, G. *Feasibility of localized immunosuppression: 2. PLA microspheres for the sustained local delivery of a soft immunosuppressant,* **Pharmazie** 2010 Jun; 65(6):429-35.

Grimstein, C.\*\*, Choi, Y-K.\*\*, Wasserfall, C., Satoh, M., Atkinson, A., Brantly, B., Campbell-Thompson, M., and **Song, S.\*** *Alpha-1 Antitrypsin Protein and Gene Therapies Decrease Autoimmunity and Delay Arthritis Development in Mouse Model.* **Journal of Translational Medicine** 2011, 9:21.

Li, H.\*\* , Zhang, B.\*\* , Lu, Y.\*\* , Jorgensen, M., Petersen, B., and **Song, S\***. *Adipose Tissue-Derived Mesenchymal Stem Cell-Based Liver Gene Delivery*. **Journal of Hepatology** 2011, 54:930-938.

Le, H. T.\*\* , Hirko, A.C., Thinschmidt, J.S., Grant, M., Li, Z. Peris, J. King, M.A. Hughes, J.A. and **Song, S\***. *The protective effects of plasma gelsolin on stroke outcome in rats*. **Experimental & Translational Stroke Medicine** 2011, Nov 2; 3(1):13. [Epub ahead of print].

Cao, J., Gregorie, BR., Sun, L., **Song, S.** *Alpha-1 antitrypsin reduces ovariectomy-induced bone loss in mice*. **Annals of the New York Academy of Sciences** 1240 (2012) E31-E35 (doi: 10.1111/j.1749-6632.2011.06370.x).

Mong-jen Chen\*\* , Yuanqing Lu\*\* , Takashi Hamazaki, Hsin-Yin Tsai\*\* , Kirsten Erger, Thomas Conlon, Ahmed S. Elshikha\*\* , Hong Li\*\* , Arun Srivastava, Chunli Yao\*\* , Mark Brantly, Vince Chiodo, William Hauswirth, Naohiro Terada, **Sihong Song\*** *Reprogramming adipose tissue derived mesenchymal stem cells (AT-MSCs) into pluripotent stem cells by a mutant AAV vector*. **Human Gene Therapy Method**, 2014, 25:72-82

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