

## SIHONG SONG

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### EDUCATION

1978-1982 **B.S. in Animal Science**, Jilin Agricultural University, Changchun, P.R. China  
1985-1989 **M.S. in Animal Science**, Jilin Agricultural University, Changchun, P.R. China  
1992-1996 **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

### PROFESSIONAL EXPERIENCE

1982-1986	<b>Teaching and Research Assistant (Faculty)</b> Department of Animal Science, Jilin Agricultural University, China.
1987-1991	<b>Lecturer (Faculty)</b> Jilin Agriculture University, China
1992-1996	<b>Graduate Assistant</b> Department of Dairy and Poultry Sciences, University of Florida.
1996-1999	<b>Post Doctoral Associate</b> Department of Molecular Genetics and Microbiology, University of Florida
1999-2001.7	<b>Research Assistant Professor</b> Department of Pediatrics, Powell Gene Therapy Center, University of Florida
2001.8-2006.6	<b>Assistant Professor</b> Department of Pharmaceutics, University of Florida College of Pharmacy
2006.7-2023.6	<b>Associate Professor</b> Department of Pharmaceutics, University of Florida College of Pharmacy
2015.5-2023.6	<b>Graduate Coordinator</b> Department of Pharmaceutics, University of Florida College of Pharmacy
2023.7-present	<b>Professor</b> 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, Asia Pacific League of Associations for Rheumatology (APLAR)
- 2009-2011 University of Florida Research Foundation (UFRF) Professorship Award.

## MEMBERSHIPS

- Member of University of Florida Genetics Institute (UFGI)
- Member of University of Florida Diabetes Institute (UFDI)
- Member of the American Society of Gene & Cell Therapy (ASGCT)
- Member of Sigma Xi, The Scientific Research Society

## COMMITTEES SERVED:

### At University of Florida:

- 2003-2007: Admission Committee of College of Pharmacy, UF  
2008-2010: Tenure and Promotion Committee of College of Pharmacy, UF  
2011-2013: Academic Performance Committee of College of Pharmacy, UF  
2013-2014: Junior Faculty Advisory Committee for Dr. Guohua An, Ph.D. Assistant Professor  
2014-2016: Curriculum Committee of College of Pharmacy, UF  
2013-2017: Junior Faculty Advisory Committee for Dr. Stephan Schmidt, Ph.D. Assistant Professor  
2014-2018: Tenure and Promotion Committee of College of Pharmacy, UF  
2017-2020: Graduate Council, UF Graduate School.  
2019: Research Assistant Professor Recruiting Committee (Chair).  
2019-2020: ACPE Self-Study Committee on Facilities and Resources  
2020: Assistant Professor Recruiting Committee.  
2021: Research Professor Recruiting Committee (Chair)  
2018-present: Admission Committee of College of Pharmacy, UF  
2015-2023: Graduate Education Committee (GEC), College of Pharmacy, UF  
2021-present Faculty Enhancement Opportunity Fund Committee (2012-2024 term), UF

### For Associations and Conferences

- 2011-2017: *The Gene & Cell Therapy of Genetic and Metabolic Diseases Committee*  
American Society of Gene & Cell Therapy  
2012 *Organizing Committee* “2<sup>nd</sup> International Conference on Pharmaceutics & Novel Drug Delivery Systems”  
2012 *Session Chair* of New Technology of Drug Delivery Systems, “2<sup>nd</sup> International Conference on Pharmaceutics & Novel Drug Delivery Systems”.

2012            *Plenary Section Chair*, II Russian congress with international participation-  
Molecular basis of clinical medicine: state-of-the art and perspectives, Saint-  
Petersburg, Russian Federation, June 18-20, 2012

**SERVED AS A GANT REVIEWER FOR:**

NIH grant, ZRG1 SSS1(12) SBIR/STTR: Cancer Diagnostic and Treatment, 2003  
Department of Health Clinician Scientist Award (DHCSA), 2005  
Italy Telethon grant, 2005.  
UF RGP grant-COP 2006, 2008, 2009  
UF RGP grant, 2010, 2013, 2020, 2021  
US-Israel Binational Science Foundation grant review, 2010.  
United kingdom Medical Research Council (MRC) grant review, 2020

**SERVED FOR SCIENTIFIC JOURNALS:**

***AS AN EDITOR FOR:***

***Biomolecules.*** Special Issue Editor for Novel Applications and Mechanism of  
Proteinase Inhibitors (2022)

***ON EDITORIAL BOARD FOR:***

***Human Gene Therapy***  
***Current Gene Therapy***

***AS A REVIEWER FOR:***

<b><i>Journal of Clinical Investigation (JCI)</i></b>	<b><i>PNAS</i></b>
<b><i>Diabetes</i></b>	<b><i>Current Gene Therapy</i></b>
<b><i>Molecular Therapy</i></b>	<b><i>American Journal of transplantation</i></b>
<b><i>Gene Therapy</i></b>	<b><i>Clinical Immunology</i></b>
<b><i>Journal of Leukocyte Biology</i></b>	<b><i>Scientific Reports</i></b>
<b><i>Respiratory Research</i></b>	<b><i>Clinical Experimental Immunology</i></b>
<b><i>Molecular Genetics and Metabolism</i></b>	<b><i>Molecular and Cellular Biochemistry</i></b>
<b><i>Nucleosides, Nucleotides &amp; Nucleic Acids</i></b>	<b><i>Tissue and Cell</i></b>
<b><i>Clinical and Experimental Rheumatology</i></b>	<b><i>International Journal of Medical Science</i></b>
<b><i>Histology and Histopathology</i></b>	<b><i>Recent Patents on Regenerative Medicine</i></b>
<b><i>PLoS ONE</i></b>	<b><i>European Journal of Pharmacology</i></b>
<b><i>Critical Care</i></b>	<b><i>Inflammation Research</i></b>
<b><i>Life Science</i></b>	<b><i>Immunological Research</i></b>
<b><i>Trends in Molecular Medicine</i></b>	<b><i>Journal of Neuroinflammation</i></b>
<b><i>Molecular and Cellular Endocrinology</i></b>	<b><i>BoiFactors</i></b>
<b><i>Laboratory Investigation</i></b>	<b><i>Chronic Obstructive Pulmonary Diseases</i></b>

**TEACHING EXPERINCE**

***At University of Florida***

2005-present (odd years);            **Pharmaceutical    Gene    Delivery    (PHA    6183,    Course**

	<b>Coordinator).</b>
2013-present (every fall):	<b>Biotechnology &amp; Drug Development in the Biopharmaceutical Industry (PHA5172, Course Coordinator).</b>
2012-presnet (even years):	<b>Molecular Therapy I – Vectors and Molecular Mechanisms (GMS 6251, two lectures for “Gene Modified Stem Cells”).</b>
2015-present (fall and spring):	<b>Graduate Seminar (PHA6938), Dept. of Pharmaceutics.</b>
2020-prestnt	<b>Special Topics: Grant Writing (PHA6939 changed to PHA6740 in 2022 summer, 1 Credit, faculty advisor for workshops)</b>
2017-2018	<b>Introduction of Graduate Studies (PHA6894, Course Coordinator).</b>
2016 Spring	<b>Introduction of Graduate Studies (PHA6894).</b>
2007-2014 (every fall):	<b>Clinical Biochemistry (PHA 5451, Course Coordinator).</b>
2003 fall -2006 fall,	<b>Clinical Biochemistry (PHA 5451).</b>
2002 fall,	<b>Clinical Biochemistry (PHA 5933).</b>
2002 fall,	<b>Graduate Seminar (Coordinator).</b>
2000 spring,	<b>Paper Discussion (GMS 6002).</b>
<b><i>At Jilin Agricultural University</i></b>	
1987-1990,	<b>Animal Nutrition,</b>
1982-1986:	<b>Animal Production</b>

## **SUPERVISED STUDENTS AND POST DOCTORAL ASSOCIATES**

### **Ph.D. Students:**

1. Mei Tang, Ph. D. student (August 2002-December 2006) “*Alpha 1 antitrypsin gene therapy and its immunoregulatory function for preventing type 1 diabetes in non-obese diabetic mouse*”.
  - Winner of poster competition: 17<sup>th</sup> Annual Research Showcase of College of Pharmacy, University of Florida, April 15, 2004.
2. Christian Grimstein, Ph. D. student (January 2005-December 2008) “*Alpha-1 antitrypsin protein and gene therapy for the prevention of rheumatoid arthritis in mouse models*”
  - Recipient of 2005 Travel Grant (Research Scholar/University of Florida)funded by Bavarian Pharmacist Foundation (Bayerische Apothekerstiftung)
  - Finalist of Oral Competition, 2006 South-East Regional Interdisciplinary Symposium (SERIS) at University of Florida, Gainesville, FL.
  - Recipient of Travel Award, Poster presentation in 2007 AAPS Biotech Section.
  - Winner of senior oral competition: 21<sup>st</sup> Annual Research Showcase of College of

- Pharmacy, University of Florida, Feb 21, 2008
- Recipient of travel award, 2008 ASGT Annual Meeting
  - Recipient of AMGEN Travel award for 2008 AAPS National Biotechnology Conference
3. Hong Li, Ph. D. student (August 2004-August 2009) “*Adult stem cell based gene therapy for alpha 1 antitrypsin deficiency*”
    - Winner of senior oral competition: 22<sup>nd</sup> Annual Research Showcase of College of Pharmacy, University of Florida, Feb 19, 2009
  4. Yan Ren, Ph.D. student (August 2006-May 2011) “*In vitro and in vivo effects of  $\alpha 7$  nicotinic receptors gene delivery on neurodegenerative pathways*”
  5. Matthias Fueth, Ph. D. student (August 2007-December 2011) “*Alpha 1 antitrypsin for the treatment of doxorubicin-induced CVD*”
    - Finalist of senior oral competition: 22<sup>nd</sup> Annual Research Showcase of College of Pharmacy, University of Florida, Feb 17, 2009
  6. Huong Thu Le, Ph.D. student (August 2007-May 2012) “*Gelsolin and AAT for the treatment of stroke in rat model*”
  7. Mong-jen Chen, Ph.D. student (August 2009-December 2013) “*Development of a novel stem cell approaches for autologous liver regeneration*”
  8. Guanming Chen, M.S. student (August 2011-December 2013) “*Protective effect of Alpha-1 Antitrypsin (AAT) on DSS-induced colitis in mouse models*”
  9. Mohammad A. Akbar, Ph.D. student (January 2010-May 2017) “*The evaluation of alpha-1 antitrypsin protein, gene, and stem cell-based therapies in animal models of osteoporosis*”
    - Nominated by University of Florida for the 2014 HHMI international Student Research Fellowship (in the final list of 12 out of >100 eligible applicants).
    - Nominated by University of Florida for the 2015 HHMI international Student Research Fellowship (in the final list of 7 out of >100 eligible applicants).
    - Recipient of the Federico Vilallonga Memorial Award (2015), UF COP.
    - 2015 UF travel award for ASGCT annual meeting.
    - 2015 travel award from AMGEN to attend the 2015 AAPS National Biotechnology Conference in San Francisco, California, June 8-10, 2015.
    - 2015 Meritorious Abstract Travel Award to attend the ASGCT 18th Annual Meeting, May 13-16, 2015, in New Orleans, LA.
    - 2015 graduate student travel award from AABPS (American Association of Bangladeshi Pharmaceutical Scientist) for The AABPS convention on August 8-9 in Philadelphia
    - Finalist in research competition, College of Pharmacy Annual Research Showcase, 2016, University of Florida (Received award plaque)
  10. Ye Yuan, Ph.D. student (August 2014-December 2019) “*Development AAT deficient mouse models*”
    - 2015 Certificate of Outstanding Academic Achievement of University of Florida
    - 2015 Charlotte M. Liberty Scholarship
    - 2016 Charlotte M. Liberty Scholarship

- 2016 Certificate of Outstanding Academic Achievement of University of Florida
  - Finalist in research competition, College of Pharmacy Annual Research Showcase, 2018, University of Florida (Received award plaque)
  - QE 2018/2.
  - 2018 Certificate of International Student Achievement Award, UF.
11. Ahmed Samir Elshikha (August 2015-August 2019) “*Development of lupus models and the treatment of lupus*”
    - Graduate School Fellowship.
    - Recipient of UF GSC Travel Grant award (Oct. 2015)
    - Recipient of Meritorious Abstract Travel Award (ASGCT 19th Annual meeting, Washington, DC, May 4-7, 2016)
    - Finalist in research competition, College of Pharmacy Annual Research Showcase, 2017, University of Florida (Received award plaque)
    - QE 2018/6.
  12. Jordan Stokes, Ph.D. Student (June 2022-Present) “*Computational design and gene delivery of novel anti-inflammatory proteins for autoimmune diseases*”
    - 2022 Board of Education (BOE) Summer Fellowship
    - McKnight fellowship

#### **Postdoctoral Associates:**

1. Yuanqing Lu, M.D., Postdoc (November 2002-2014) “*Prevention of type 1 diabetes and stem cell biology*”.
2. Young-Kook Choi, Ph. D., Postdoc, (April 2003-December 2007) “*AAV vector Biology and gene therapy*”.
3. Bin Zhang, Ph. D. (May 2004- March 2009) “*Islet Biology and AAT gene expression and regulation*”
4. Christian Grimstein, Ph.D. (Jan 2009-July, 2009) “*Anti-inflammatory functions of AAT*”
5. Hong Li, Ph.D. (Sept. 2009-Nov. 2009) “*Stem cells transplantation*”
6. Ahmed Samir Elshikha, Ph.D. (Jul 2020-Present) “*Development of a novel gene therapy for lupus*”
  - 2022 Poster winner in 35<sup>th</sup> UF college of Pharmacy Research Showcase
  - 2022 AAI Travel Award.

#### **Visiting Professor, Students and Scientists:**

1. Hongxia Ma, Ph. D. (Associate professor in Pharmacology from Jilin Agricultural University, Changchun, China, August 2007-September 4 2008, supported by China Scholarship Council ) “*Effect of hAAT on gene expression in islet cells and macrophages*”, “*Prevention of T1D using Tet-On-hAAT system*”, “*Prevention and reversal of T1D by hAAT*”
2. Weidong Li, MD. (PhD student from Beijing University of Chinese Medicine, China, Sept 1, 2008 to May 1, 2009, supported by China Scholarship Council) “*Pilot studies of AAT for colitis and cardiovascular diseases* ”
3. Chunli Yao, M.D., Ph.D. (Associate Professor from Jilin University, September 2008-

- February 2009) “*Stem cell transplantation*”
4. Tianqi Zhu, (Undergraduate student from Fudan University, China, March-June, 2011)
  5. Yue Zheng, (Undergraduate student from Fudan University, China, March-June, 2011)
  6. Junling Qing (Ph.D. student from Jilin University, China, September, 2011-March 2013, supported by China Scholarship Council) “*Development of rAAV vaccines for infectious diseases*”
  7. Tianwei Guo (Ph. D. student from Beijing University of Chinese Medicine, China, Sept, 2012 to August, 2013, supported by China Scholarship Council) “*AAT for IBD*”
  8. Ahmed Samir Elshikha (Ph.D. student from Zagazig University, Egypt, supported by Egyptian Government Scholarship, February 2013-January 2015; 2015/2-2015/8 OPS). “*AAT for lupus*”
    - Finalist of Junior Oral competition: 27<sup>th</sup> Annual Research Showcase of College of Pharmacy, University of Florida, Feb 20, 2014.
    - Winner of Graduate Student Poster Competition, 28<sup>th</sup> Annual Research Showcase of College of Pharmacy, University of Florida, Feb 19, 2015.
  9. Wael Abd-El Halim Hegazy, Ph.D. (Assistant Prof. of Microbiology and Immunology, Faculty of Pharmacy, Zagazig University, Egypt; supported by scholarship of Egypt Government, Feb. 12, 2014 – August 31, 2014) “*TALLEN for targeting mouse AAT*”
  10. Xin Guo, Ph.D. (March, 2015-March 2016) from Southwest University, Chongqing, P.R. China and supported by China Scholarship Council. *Pharmacy teaching*
  11. Eslam Elsayed, MS., (July 25, 2016-January 24, 2017) from Microbiology and Immunology Dept., Faculty of Pharmacy, Zagazig University, Egypt, supported by scholarship of Egypt Government. *MSC for bone*
  12. Shaimaa Rezk, MS., (August 24, 2016-August 23, 2018) from Microbiology and Immunology Dept., Faculty of Pharmacy, Zagazig University, Egypt, supported by scholarship of Egypt Government. *Construction of CRISPR for AAT gene targeting*
  13. Hong Yan, Ph.D. (November 2016-November 2018) from Hunan University of Chinese Medicine. *Drug targeting*.
  14. Shalmali Gadgil, Summer Intern (June 14-September 13, 2021), MS student from University of Aberdeen, UK.
  15. Iram Hyder, visiting scholar (November 2022 to present)

#### **Pharm. D. Students:**

1. Keith Lowe, 2<sup>nd</sup> year Pharm. D. student, recipient of 2003-2004 Merck Research Scholar Program Award (Adviser, May 2003-August 2004) “*Construction and In Vitro Evaluation of a Noval AAV vector for Type I Diabetes Mellitus*”.
2. Yitsung Wang, 1<sup>st</sup> year Pharm. D. student, recipient of a NIH fellowship award (NIH T35-HL07489, Adviser, May 2005-August 2005) “*Construction and Evaluation of Regulatable Gene Therapy for Type I Diabetes*”
3. Tina Chen, 1<sup>st</sup> year Pharm. D. student, recipient of 2012 Summer Research Internship from the University of Florida, College of Pharmacy. “*rAAV stability*”
4. Tina Chen, 2<sup>st</sup> year Pharm. D. student, recipient of 2013 Summer Research Internship from the University of Florida, College of Pharmacy. “*Generating rAAV vectors for Dengue Vaccine*”

5. David Nardo, 2<sup>st</sup> year Pharm. D. student, recipient of 2014 Summer Research Internship from the University of Florida, College of Pharmacy.
6. Ted Lee, 2<sup>nd</sup> year Pharm. D. student, 2014 Summer Research Credit.
7. Eduardo Diaz, 1st year Pharm. D. student, recipient of 2015 Summer Research Internship from the University of Florida, College of Pharmacy.
8. Joshua L Allman,
  - a. 2016 Fall Research Credit. 2<sup>nd</sup> Year Pharm. D. student.
  - b. 2017 Independent Summer Research Program (ISRP).
9. Hernando Santacoloma, (5/17-7/16, 2021) 2021 Independent Summer Research Program (ISRP)

**German (or European) Pharmacy Exchange Students:**

1. Andreas Niclas Foerster (March – Sep. 2002) “*Infection of five serotype of AAV vector to culture cells*”.
2. Vera Wallmeier (April – Oct. 2002) “*estimation of efficiency of rAAV integration in vitro*”
3. Thomas Gardemann (January 2003-July 2003) “*In vitro and in vivo detection of elafin and construction of dsAAV vector*”.
4. Holger Piekuth (May 1, 2003-October 31, 2003) “*Construction of dsAAV vectors*”.
5. Katharina (Ina) Manzer (May 1, 2003-October 31, 2003) “*Construction of dsAAV vectors*”.
6. Christian Grimstein (May 12, 2004-October 12, 2004) “*Construction of scAAV vectors*”.
7. Miriam Siemer (November 14, 2005 – May 14, 2006) “*Development of rAAV vectors for dynorphin gene delivery*”
8. Kay Werner Klindwort (May 1, 2006-October 31, 2006) “*Dynorphin gene delivery*”
9. Astrid Holzinger (November 2, 2006-May 1, 2007) “*scAAV vectors for Dynorphin gene therapy*”
10. Katharina Tomala (June 10, 2007- December 10, 2007) “*Salivary delivery of rAAV5-DYN and rAAV5-CB-hAAT for pain relieve*”
11. Benjamin Ma (January 14, 2008-July 2008) “*Development of serpin expression vectors*”
12. Anna Ullrich (January 14, 2008-July 2008) “*Development of mutant AAT expression vectors*”
13. Sojia Rothweiler (January 1, 2009 to March 1, 2009) “*Effect of AAT on cathepsin S activity*”
14. Linda Theisen (January 1, 2009 to June 1, 2009) “*Construction of rAAV vectors for immune-tolerance therapy*”
15. Peggy Fungke (November 1, 2008 to June 1, 2009) “*Gene expression in hypothalamus*”
16. Katja Giersch (May, 2009 to Nov, 2009) “*Construction of hAAT gene correction vectors*”
17. Cornelia Legler (January 2010 to June 2010) “*Alzheimer’s disease models*”
18. Afshin Sobhi (May 2010-Nov 2010)
19. Lonneke Maria Erkelens (September 2010-March 2011), from University of Utrecht, Netherlands. “*Regulated transgene expression in myoblasts and the prevention of lung hemorrhage by AAT*”
20. Godelieva Ponjee (September 2010-March 2011), from University of Utrecht, Netherlands. “*Interaction of hAAT and doxorubicin, and application of AAT for lupus*”
21. Laura Herlan (March 2011-September 2011) “*Protection of cardiomyopathy in rat model*”
22. Christian Granwitz (December 2011-May 2012) “*Construction of rAAV vectors for dengue*”

*vaccine*”

23. Jesper Spoelder (Feb. 15 2012-August, 2012), from University of Utrecht, Netherlands. “*AAT for cancer*”
24. Stephanie Marie-Christina Larissa Hinske (May 2, 2012-November 2012), University of Muenster, Germany. “*Stem cell differentiation*”
25. Andrea Ritter (May 1, 2013-Nov. 1, 2013), from Bayerische Julius-Maximilians-Universität Würzburg, Germany. “*Detection of mouse AAT*”
26. Rasha Hassan (Nov. 1, 2013-May 1, 2014) “*Expression of a mutant human AAT (d6-hAAT) in 293 cells*”.
27. Claire Bigot (Much 4 2014-Sep. 4, 2014) “*Effect of AAT on osteoclast formation*”
28. Henrike Charlotte Plate (May, 2016-Nov, 2016) from Westfälische Wilhelms-University. “*Detection of mouse AATs*”
29. Tanja Hoffmann (Nov. 2, 2016 to May 1, 2017) from University of Regensburg “*Detecting mouse AAT, human AAT antibodies in lupus mice*”
30. Hedwig Wolny (Nov. 2, 2016 to May 1, 2017) from University of Regensburg “*Detecting cytokines in senescent cells and hAAT in gene targeted cells*”
31. Clara Sophia Mundry (Dec.1, 2017 to May 31, 2018) from Johann-Wolfgang-Goethe-University, Frankfurt.
32. Carolin Arnold (April 29, 2022-November 1, 2022) from Germany

#### **Ph.D. Rotation Students (IDP-Interdisciplinary Program, G/G-Genetics & Genomics):**

1. Yi Hua (Fall, 1998, IDP) “*Construction and evaluation of rAAV vectors for regulated transgene expression*”.
2. Nikki Rhodin (Spring, 1999, IDP) “*In vivo evaluations of regulated transgene (IL-4 and IL-10) expression in myoblast cells (C2C12)*”
3. Thomas Conlon (1999-2001, IDP) “*Liver-directed gene delivery for alpha-1-antitrypsin deficiency*”.
4. Jessica Stone (Fall 2013, G/G) “*Exploring the Role of Irisin*”

#### **Post-Baccalaureate Students:**

1. Claudia L. Sotillo (BS, NIDDK supported minority student, Januray 2009 to Jun 2010) “*AAT dificeint mouse model and correction of the mutant gene*”

#### **Under graduate students:**

1. Kevin Foust, (1999-2001) “*Characterization and identification of the promoter of human translation elongation factor 1 $\alpha$ 2 (EF-1 $\alpha$ 2) gene for muscle-directed gene delivery*”.
2. Chris Porter, (2000-2001), “*In vitro and in vivo analysis of GT-enhancer activities*”.
3. Elaine Liu, (2004-2005), “*In vitro evaluations of transgene expression*”
4. Xueying Wang (2007-2008), recipients of University Scholar and HHMI Undergraduate Research Award “*Alpha 1 antitrypsin gene therapy for the treatment of rheumatoid arthritis*”
5. Chia-Wei Tsai (2006 fall-2008 Spring) “*Evaluation of mutant human alpha 1 antitrypsin (hAAT for their anti-enzymatic activities*”
6. Abigail Danos (2017 fall- 2018 Summer). “*AAT functional evaluations*”
7. Cameron Courtney (2023 Spring-present) URSP-recipient, Pre-Med.

**High School Students:**

1. Frankie Beauliey (June 11-July 29, 2006), Student Science Training Program at the University of Florida.

**Graduate Committees:**

Served as a Committee Chairman:

1. Mei Tang, 2006/12 Ph.D., Department of Pharmaceutics, College of Pharmacy.
2. Christian Grimstein, 2008/12 Ph.D., Department of Pharmaceutics.
3. Hong Li, 2009/8 Ph. D., Department of Pharmaceutics.
4. Yan Ren, 2011/5 Ph.D., UF Genetics Institute
5. Matthias Fueth, 2011/12 Ph.D., Department of Pharmaceutics.
6. Huong Le, 2012/5 Ph.D., Department of Pharmaceutics.
7. Mong-jen Chen, 2013/12 Ph.D. student, Department of Pharmaceutics.
8. Guanming Chen, 2013/12 M.S. student, Department of Pharmaceutics
9. Mohammad A. Akbar, 2017/5 Ph.D, Department of Pharmaceutics
10. Ye Yuan, 2019/12 Ph.D., Department of Pharmaceutics
11. Ahmed Elshikha, 2019/8 Ph.D. student, Department of Pharmaceutics
12. Jordan E. Stokes, 2022/8 Ph.D. student, Department of Pharmaceutics

Served as a Committee Member:

1. Yan Gong Whisler, 2004/5 Ph.D., Department of Pharmaceutics.
2. Hao Zhu, 2004/8 Ph.D., Department of Pharmaceutics.
3. Ke Ren, 2005/12 Ph.D., Department of Pharmaceutics.
4. Gregory G. Simon, 2005/12 Ph.D., IDP program, College of Medicine.
5. R. Fleeter Schwartz, 2005/12 M.S., Department of Pathology
6. Todd Brusko, 2006/8 Ph.D., IDP program, College of Medicine.
7. Aaron Hirko, 2006/8 Ph.D., Department of Pharmaceutics.
8. Changqing Li, 2006/12 Ph.D., Dept. of Biomedical Engineering.
9. Wouter Driessen, 2007/5 Ph.D., Department of Pharmaceutics.
10. Nathalie Toussaint, 2007/5 Ph.D., Department of Pharmaceutics.
11. Leah Villegas, 2007/8 Ph.D., Department of Pharmaceutics.
12. Patricio Tapia, 2007/12 Ph.D., Dept. of Civil & Coastal Engineering
13. Matthew Parker, 2008/12, Ph.D. IDP program, College of Medicine.
14. Lindsey M. Bierschenk, 2009/5 MS. Department of Pathology.
15. Ryan Mesaros, 2010/5 M.S., Department of Pharmaceutics.
16. Yanfei Qi, 2009/12 Ph.D., Department of Pharmacodynamics.
17. Vinayak Shenoy, 2009/12 Ph.D., Department of Pharmacodynamics.
18. MyPhoung Le, 2010/5 Ph.D., Department of Pharmaceutics.
19. Michael Weide, Ph.D. student (left without degree), Department of Pharmaceutics.
20. Yao Sun, 2010 Ph.D/8., Dept. of Biomedical Engineering.
21. Brett Howell, 2010/5 Ph.D., Department of Chemical Engineering
22. Courtney Myhr, 2011/5 MS., Department of Pathology.
23. Fuhua Chen, 2012/12 Ph.D., Department of Mathematics.
24. Ruixin Jiang, 2012/12 Ph.D., Dept. of Biomedical Engineering.
25. Lei Xi, 2012/12 Ph.D., Dept. of Biomedical Engineering.

26. Kai Xiao, 2012/12 Ph.D., Department of Medicine.
27. Zachary Watson, 2013/12 Ph.D., IDP program, College of Medicine.
28. Erin Bruce, 2013/12 Ph.D. Department of Pharmacodynamics.
29. Young Mee Yoon, 2014/8 PH.D. IDP program, College of Medicine.
30. Amanda Posgai, 2014/12 PHD, IDP program, College of Medicine.
31. Anandharajan Rathinasabapathy, 2015/8 PhD, Department of Pharmacodynamics.
32. Robert (Chad) Water, 2015/8 PhD, Department of Pharmaceutics.
33. Nivea Falcao, 2015/8 PhD, Department of Pharmaceutics.
34. Alexander Voelkner, 2015/12 Ph.D., Department of Pharmaceutics.
35. Di Bei, 2015/12 Ph.D., Department of Pharmaceutics.
36. Jessica Stone, 2014/8-2015 MS, UF Genetics Institute.
37. Ing-orn Prasanchaimontri, 2017/5 PhD Department of Pharmaceutics (QE5/2015).
38. Syed Saoud Zaidi, 2017/8 Ph.D. Department of Pharmaceutics (QE 12/2016).
39. Marc Tobias Heinrichs, 2017/12 Ph.D. Department of Pharmaceutics. (QE11/21/2016)
40. Vipada Khaowroongrueng, 2018/8 Ph.D. Department of Pharmaceutics. (QE 11/28/2016)
41. Abhigyan Ravula, 2018/5 Ph.D. Department of Pharmaceutics (2016-12 QE)
42. Yichao Yu, 2018/8 Ph.D. Pharmaceutical Sciences.
43. Jie (Joy) Shao, 2019/12 Ph.D., Department of Pharmaceutics (QE 3/2018).
44. Lu Yang, 2020/12 Ph.D. Department of Chemistry (QE 2-2019).
45. Saima Subhaini, Ph.D. student, Pharmaceutical Sciences. (until 2019/9)
46. Nasser Nassiri Koopaei, 2020/12 Ph.D. student, Department of Pharmaceutics (QE 2019/12).
47. Xiang Li, 2023/12 Ph.D., Department of Chemistry (Tan/Bucher Group, QE 4/23/2021)
48. Jiatong Guo, 2019/5 MS, Department of Chemistry (Guo Group).
49. Jiatong Guo, 2023, Ph.D., Department of Chemistry (Guo Group).
50. Evelyn Franco, 2022/8 Ph.D., Department of Pharmaceutics (Brown Group).
51. Long Nguyen, 2023/5 Ph.D., Department of Chemical Engineering (Piyush Jain Group QE 2020/5).
52. Gamze Bulut, 2023/12 Ph.D. Department of Pharmaceutics (Schmittgen Group).
53. Yi-Hua Chiang, Ph.D. Student, Department of Pharmaceutics (He Group, Changed).
54. Zijing Xu, Ph.D. Student, Department of Pharmaceutics (Zhang Group).
55. Kevon Jolly, Ph.D. Student, Department of Pharmaceutics (Zhang Group).

## **INVITED AND ORAL PRESENTATIONS:**

### **National and International:**

1. “*Ex Vivo Gene Transfer and Transplantation of Liver Stem Cell for Alpha-1 Antitrypsin Deficiency*”. Sixth Annual Meeting of American Society of Gene Therapy, Washington DC, May, 2003. [https://doi.org/10.1016/S1525-0016\(16\)40472-7](https://doi.org/10.1016/S1525-0016(16)40472-7)
2. “*Effect of DNA-PK on rAAV2 Persistence in Mouse Liver*”. Sixth Annual Meeting of American Society of Gene Therapy, Washington DC, May, 2003. [https://doi.org/10.1016/S1525-0016\(16\)40848-8](https://doi.org/10.1016/S1525-0016(16)40848-8)
3. “Effect of hepatocyte division on rAAV integration”. Xth Parvovirus Workshop, St. Petersburg, FL, September 11, 2004.

4. "Alpha 1 antitrypsin gene therapy for type 1 diabetes" The First International Conference of Chinese Physiological Scientists-Physiological Sciences in the Postgenomic Era. July 14-17, 2004, Beijing.
5. "*AAV mediated AAT gene therapy for autoimmune diabetes*" XIth Parvovirus Workshop, Les Diablerats, Switzerland, August, 27-31, 2006
6. "*Alpha 1 Antitrypsin (AT) for the Prevention of Type 1 Diabetes*", 6<sup>th</sup> International Scientific Conference New Insights into the Biology of AAT: The Expanded Role of AAT in the Treatment of AAT Deficient Individuals and Other Diseases, Coral Gables, Florida, Feb 8-10, 2007.
7. "*rAAV Vectors for Gene Therapy of Type 1 Diabetes*", 6<sup>th</sup> Symposium of New Development in Clinical Pharmacy and Clinical Pharmacology, June 9, 2007, Munich, Germany.
8. "*Gene therapy and its potential for the treatment of cancer*" July 3, 2008 China Medical University, Shenyang, China
9. "*rAAV vectors for gene delivery*" July 15, 2008, Jilin Agricultural University, Changchun, China
10. "*Alpha 1 antitrypsin (AAT) for rheumatoid arthritis*", September 25, The 13<sup>th</sup> Congress of the Asia Pacific League of Associations for Rheumatology (APLAR 2008), Yokohama, Japan.
11. "*Alpha 1 antitrypsin (AAT): functions and its therapeutic potentials*" May 4, 2009, Beijing University of Chinese Medicine
12. "*Alpha 1 antitrypsin for the treatment of autoimmune diseases*", Oct 3-5, 2008, 2<sup>nd</sup> World Conference on Magic Bullets (Ehrlich II), Nurnberg, Germany
13. "*Functional studies of AAT: progress in developing of AAT deficient mouse model*" Oct. 15, 2010, Alpha 1 Foundation Investigators Meeting, Miami, USA.
14. "*Recombinant Adeno-Associated Virus (rAAV) Vectors for Gene and Cell Therapy*" April 25, 2011, Shandong University, Jinan, China.
15. "*Recombinant Adeno-Associated Virus (rAAV): Host and Vector Interactions*" May 6, 2011, Institute of Microbiology, Chinese Academy of Sciences, Beijing, China.
16. "*Therapeutic potentials of AAT for diseases associated with autoimmunity and inflammation*" 12<sup>th</sup> Gordon L. Snider Critical Issues Workshop "New formulations and application of alpha-1 antitrypsin, June 24, 2011, Bethesda, MD.
17. "*Alpha 1 antitrypsin Therapy for autoimmune diseases*". The 6<sup>th</sup> International Symposium on the Chemistry and Biology of Serpins, Oct. 23-26, 2011, Chapel Hill, NC.
18. "Gene Based Protein Drug Delivery" Keynote Speaker, 2<sup>nd</sup> International Conference on Pharmaceutics and Drug Delivery Systems", Feb. 20-22, 2012, San Francisco, CA

19. “Gene Drug: Challenges and Hope” Honorable Guest Speaker, 2<sup>nd</sup> International Conference on Pharmaceuticals and Drug Delivery Systems”, Feb. 20-22, 2012, San Francisco, CA
20. “Stem cell Based Protein Drug Delivery”, Invited Speaker in Track: New Technology of Drug Delivery Systems, 2<sup>nd</sup> International Conference on Pharmaceuticals and Drug Delivery Systems”, Feb. 20-22, 2012, San Francisco, CA.
21. “Gene and stem cell therapies for genetic diseases”, plenary lecture, II Russian congress with international participation-Molecular basis of clinical medicine: state-of-the art and perspectives, Saint-Petersburg, Russian Federation, June 18-20, 2012.
22. “Potential therapeutic applications of alpha 1 antitrypsin (AAT) – A multifunctional protein” Jilin University College of Medicine, Changchun. Sept. 14, 2012.
23. “A novel therapy for controlling autoimmunity and inflammation” Institute of Radiation Medicine of Chinese Academy of Medical Sciences & Peking Union Medical College, Tsinghua University, Tianjin, China. Sept. 17, 2012.
24. “Development of novel therapies for the treatment of autoimmune diseases” Keynote Speaker, 3<sup>rd</sup> International Moscow’s Conference-Immunophysiology: “Autoimmunity in health and disease through the light and view of predictive and preventive medicine”, Moscow, Russia. October 1-3, 2012.
25. “Experience in writing research articles”, Jilin University, Changchun, China. July 6, 2013.
26. “Therapeutic potential of AAT for the treatment lupus”, Jilin University, Changchun, China. July 19, 2014.
27. “Current Advances in Application of AAT” Jilin University, Changchun, China. August 15, 2015.
28. “Controlling inflammation in multiple disease models” Jilin University, Changchun, China. Jun 10, 2016.
29. “The protective effect of Prolastin C for the treatment of lupus”, Investigator Sponsored Research Forum, Research Triangle Park, NC, July 12, 2016.
30. “*AAT therapy for autoimmune disorders*”. The 3<sup>rd</sup> International Research Conference on Alpha-1 antitrypsin, Lisbon, Portugal, April 6<sup>th</sup>, 2017. (Panelist).
31. “*Functional Study of AAT: Recent Advances*” Jilin University, Changchun, China. Jun 2, 2017.
32. “*Development of anti-inflammatory therapy*” Jilin Agricultural University, Changchun, China. Jun 3, 2017.
33. “*Therapeutic strategies for autoimmune diseases*” Beijing University of Chinese Medicine, June 9, 2017.
34. “*Current understanding of SERPIN and strategies to circumvent host immune response*” Jilin Agricultural University, Sept. 7, 2018.

35. “*Challenges and Opportunities in Gene Therapy Research*” Jilin Agricultural University, Sept. 8, 2018.
36. “*Recombinant Adeno-Associated Virus Vector (rAAV) for gene therapy*”, AstraZeneca, August 19, 2019.
37. “*Development of Gene Therapy for Autoimmune Diseases*” Horae Gene Therapy Center University of Massachusetts Chan Medical School, Feb 3, 2022.
38. “*Control of autoimmunity by alpha-1 antitrypsin: Gene therapy and beyond*” Keynote Speaker, World Pharma 2022-Global Virtual Summit on Pharmaceutical and Novel Drug Delivery Systems, August 18-19, 2022.
39. “*The protective effects of alpha 1 antitrypsin (AAT) in lupus models*”. SERPINS 2022-23 Research Seminar Series, Jan 11, 2023.

#### **Institutional:**

1. “*Adeno-associated virus (AAV) vector mediated gene delivery for alpha-1-antitrypsin deficiency*”. January 28, 2000, Animal Molecular and Cell Biology Seminar Series, University of Florida
2. “*Recombinant Adeno-Associated Virus (AAV) Vector for Gene Therapy*”, National Advisory Board Meeting , Best Western Gateway Hotel, Gainesville, Sep. 29, 2001
3. “*Effect of DNAA-PK on the molecular fate of the rAAV2 genome*”, College of Medicine, May 1, 2002
4. “*Gene Therapy for Type I Diabetes*”, University of Florida, March 21, 2003.
5. “*Recombinant Adeno-Associated Virus (rAAT) Vector Mediated Gene Delivery for Preventing Type I diabetes*” October 8, 2003, Interdisciplinary Reproductive Biology Group Seminar, University of Florida.
6. “*Therapeutic Potential of Alpha 1 Antitrypsin (AAT) for Type I Diabetes and Rheumatoid Arthritis*” August 31, 2007, Pharmacy Practice, University of Florida.
7. “*Differential immune responses to hAAT in animal models*” September 19, 2007, Pulmonology, University of Florida.
8. “*The therapeutic potential of alpha 1 antitrypsin for autoimmune diseases*”. October 5, 2007, Animal Molecular and Cell Biology Seminar Series, University of Florida.
9. “*Recombinant Adeno-Associated Virus (rAAV) Vectors for Gene and Cell Therapy*” September 1, 2010. UF Gene Therapy Center.
10. “*Effect of DNA-PK on rAAV persistence and replication*”. March 16, 2011, UF Gene Therapy Center.
11. “*Lesson from using rAAV: simple, complex or opportunity?*”. November 20, 2015, Powell Gene Therapy Center and The Division of Cellular and Molecular Therapy Department of Pediatrics.

12. “*Therapeutic Applications of Alpha 1 antitrypsin (AAT)*”. Department of Physiology and Functional Genome, College of Medicine, University of Florida. August 26, 2019

## FUNDING RECEIVED

### *SUMMARY TABLE*

Recourses	As a PI (DC+IDC)	As a Co-I (DC+IDC)
Federal grants	\$ 2,740,458	\$2,934,428
Industrial grants	\$ 892,042	\$0
Foundation Grants	\$704,923	\$84,745
Others	\$369,578	
<b>Total=\$7,996,174</b>	<b>\$4,977,001</b>	<b>\$3,019,173</b>

### *ONGOING:*

1. 1R01 DK123078-01A1 (PI=Alli)  
July 1, 2020-June 30, 2025 \$1,677,500  
“The circadian clock protein BMAL and post-translational regulation of ENaC in the kidney”  
The overall goal of this project is to investigate the physiological role of the circadian protein BMAL1 in the control of arterial blood pressure through regulation of the expression and activities of renal epithelial sodium channel (ENaC) and myristoylated alanine-rich C-kinase substrate (MARCKS).  
Role: Co-Investigator (10% effort).
2. DOD PR210731 (PI=Song)  
Feb 3, 2022-Feb 2, 2024 \$305,000  
“*Computationally Designed IL-10 Mutants for the Treatment of Inflammatory Bowel Diseases*”  
The goal of this study is to develop a more effective and safer therapy for the treatment of inflammatory bowel disease (IBD).  
Role: PI (8% effort)

### *COMPLETED:*

1. Sarepta’s (ISIP) AGR00018125, (PI=Song)  
June 30, 2020-June 29, 2023 \$762,457  
“Development of a novel gene therapy for lupus”  
The goal of this study is to develop a novel gene therapy for the treatment of systemic

lupus erythematosus (SLE, or lupus).  
Role: PI (30%).

2. UF COP PROSPR (PI=Song)  
Jan 3, 2022-Jan 2, 2023 \$20,000  
“A pilot Study- Novel molecules to target autoimmunity in rheumatoid arthritis”  
The goal of this study is to develop a novel gene therapy for the treatment of rheumatoid arthritis (RA) and a novel strategy for targeting autoimmunity.  
Role: PI (15% effort)
3. Miscellaneous Donors (PI=Song)  
August, 2012-July 2020 \$200,000  
“*Stem cell based gene therapy for AAT deficiency*”
4. AGTC, Inc.(PI=Song)  
May 2018-November 2018 \$25,085  
“*Testing of AAV-hAAT Vectors in Mice*”
5. UF COP-PROSPER fund (PI=Song)  
July, 2018-Jun, 2019 \$30,000  
“*Development of a novel toleragenic vaccine for T1D*”
6. Scholarship Award for Shaimaa Rezk from Egypt Government (PI=Song)  
Aug. 2016-Jul. 2017 \$10,000  
Aug. 2017-Jul. 2018 \$5,000  
“*Targeting of AAT gene*”
7. UF-OF (PI=Song)  
June 1, 2015-May 31, 2017. \$62,961  
“*Alpha 1 antitrypsin for the treatment of osteoporosis*”
8. Grifols Inc., Research Grant (PI=Song)  
2015-2017 \$135,000  
“*The protective effect of Prolastin C for the treatment of lupus*”
9. Scholarship Award for Ahmed Samir Elshikha from Egypt Government (PI=Song)  
Feb. 2013-Feb. 2014 \$10,000  
Feb. 2014-Feb. 2015 \$9,500  
“*Effect of AAT on lupus in animal models*”
10. UF-OF (PI=Song)  
May 1, 2012-April 2014. \$88,000  
“*Alpha 1 antitrypsin for the treatment of inflammatory bowel diseases (IBD)*”
11. Juvenal Diabetes Research Foundation (PI= George S. Eisenbarth,)  
June 1, 2011 to May 31, 2012. \$70,000 (Subcontract)  
“*Structure guided small molecule targeting of anti-insulin primary trimolecular complexes*”  
Role: Co-Investigator

12. UF-RGP, 2009 Research Opportunity Fund (PI=Song)  
May 1, 2009-April 30, 2011                      \$80,000  
"Alpha 1 antitrypsin for treatment of lupus"
13. NIDDK, P01-DK58327 (Supplemental grant to Project 2, PI=Song)  
Dec 1, 2008-July 31, 2011.                      \$200,650
14. Alpha One Foundation research grant (PI=Song)  
July 1, 2008-June 30, 2011                      \$129,923  
"Development of AAT deficient mouse models"
15. NIH, P01-AG10485 (PI of the P01=Simpkins, PI of the project=Song since 2010)  
June 15, 2007-Janary 31, 2011                      \$570,017  
"Discovery of Novel Drugs for Alzheimer's Disease"  
Role: Project PI.
16. UF-RGP opportunity fund (PI=Song)  
May 1, 2006-April 30, 2008                      \$74,117  
"Alpha 1 antitrypsin for the treatment of rheumatoid arthritis"
17. NIDDK, P01-DK58327 (PI of the P01=Byrne; PI of the project 2=Song)  
August 1, 2005- July 31, 2011.                      \$5,819,616 (Song=\$1,015,923)  
"Recombinant AAV for correction of genetic abnormalities"  
Project 2: Adult stem cell as a platform for liver gene therapy"
18. NHLBI, R21 HL079132 (PI=Song)  
July 1, 2005-June 30, 2008.                      \$354,540  
"Gene Therapy for Correction of PiZ Mutation".
19. NIH R01 HL69877 (PI-Flotte; Co-Investigator=Song)  
April 1, 2003-March 31, 2007.                      \$1,265,928  
"Preclinical & Phase I/II Trials of AAV-AAT Vectors"
20. NIH T35-HL07489, a fellowship award to Yitsung Wang (Advisor: Song),  
May 9, 2005 to August 5, 2005.                      \$4,328  
"Construction and evaluation of regulatable gene therapy for type 1 diabetes mellitus"
21. Merck Research Scholar Program Award (PI-Song, recipient Keith Lowe)  
May 2003-July 2004                      \$5,500  
"Construction and In Vitro Evaluation of a Noval AAV vector for Type 1 Diabetes Mellitus"
22. NIDDK R21 DK062652-01 (PI=Song)  
July 1, 2002-June 30, 2005.                      \$290,000  
"Anti-inflammatory Serpin (AAT and AAV) gene Transfer to Enhance Islet Transplantation".
23. Juvenal Diabetes Research Foundation 1-2002-783 (PI=Song)  
July 1, 2002-July 1, 2005.                      \$450,000 (\$150,000/year, for 3 years)  
"Anti-inflammatory Serpin Gene Therapy for Preventing Type 1 Diabetes"

24. March of Dimes Birth Defects Foundation (PI=Laipis, Co-PI=Song)  
 July 1, 2002-June 30, 2005 \$84,745/year  
*"Gene Therapy Approaches to the Treatment of Phenylketonuria (PKU): Prevention of Maternal PKU Syndrome with rAAV Vectors Expressing Phenylalanine Hydroxylase"*
25. 2002 University of Florida Research Opportunity Fund (PI=Song).  
 May 1, 2002-April 30, 2003 \$30,000  
*"Gene Therapy for Type 1 Diabetes"*
26. The JDRF gene Therapy Center for Diabetes and Diabetic Complication at the University of Florida and University of Miami.  
 May 1, 2002-May 1, 2003 \$5,000  
*"A Pilot study for Diabetes"*
27. Young Investigator Fellowship Award from Alpha One Foundation (PI=Song).  
 July, 2001-July, 2002. \$50,000  
*"Adeno-Associated Virus (AAV) Vectors for Skeletal Muscle Mediated Gene Therapy for Alpha-1 Antitrypsin (AAT) Deficiency-Preclinical Study in Non-human Primates"*.
28. 2001 Children's miracle Network Fall Award (PI=Song)  
 January, 2002- January, 2003. \$20,000 (equipment only)  
*"Alpha-1 Antitrypsin Gene Therapy for Juvenile Diabetes"*.

## PATENT

1. Flotte, T.R., **Song, S.**, Byrne, B., Morgan, M. *Materials and Methods for Gene Therapy*.  
 This patent has been licensed to Applied Genetic Technologies Corporation (AGTC) and issued in following countries:

Issued Countries	Patent No.	Issue Date
United States	US 6,461,606	10/8/2002
Austria	1071806	6/16/2004
Belgium	1071806	6/16/2004
Canada	2,326,327	6/16/2009
Cyprus	1071806	6/16/2004
Denmark	1071806	6/16/2004
Finland	1071806	6/16/2004
France	1071806	4/30/2007
Germany	1071806	6/16/2004
Great Britain	1071806	6/16/2004
Greece	1071806	6/16/2004
Hong Kong	1035205	4/25/2005
Ireland	1071806	6/16/2004
Italy	1071806	6/16/2004
Luxembourg	1071806	6/16/2004
Monaco	1071806	6/16/2004

Netherlands	1071806	6/16/2004
New Zealand	507308	10/3/2000
Portugal	1071806	6/16/2004
Spain	1071806	6/16/2004
Sweden	1071806	6/16/2004
Switzerland	1071806	6/16/2004

2. Brantly, M., Xiao, K., Levites, Y., Wang, L., **Song, S.**, Oshins, R.A., Rouhaini F.N. *Novel single chain antibody reduced mutant alpha-1 antitrypsin aggregation and toxicity*. U.S. provisional Patent Application Docket No. UF. 1098P (UF#-14434).
3. Song, S. Yuan, Y. *Targeting mouse alpha-1 antitrypsin genes by transcription activator-like effector nucleases*. U.S. provisional Patent Application Number 62118210 (UF#-15602).

## PUBLICATIONS

URL: L: <https://www.ncbi.nlm.nih.gov/myncbi/16UsiVEhSlAEdD/bibliography/public/>

(\*\* trainees in Song's lab, \* Song as a corresponding author)

### Peer Reviewed Journal Articles:

1. 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.
2. **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.
3. 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.
4. 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.
5. **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.
6. 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.

7. 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.
8. 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.
9. **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.
10. **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.
11. 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.
12. 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.
13. **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
14. Laipis PJ, Charron CE, Ross K, Reyes L, Alexander JJ, **Song S**, Steele HA, Berns KI, Zori R, Flotte TR. 2002. *Long-term correction of phenylketonuria in an animal model by recombinant AAV-based gene therapy.* **J Inher Metab Dis** 25: 615-616.
15. 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<sup>+</sup> CD25<sup>+</sup> 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.  
DOI: <https://doi.org/10.4049/jimmunol.171.5.2270>
16. 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.

17. 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
18. **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.
19. **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.
20. 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).
21. 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.
22. **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.
23. 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
24. 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
25. 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
26. 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* 2006, 25(1):6-18.

27. Petrache, I., Fijalkowska, I., Zhen, L., Medler, 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.
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2. Ahmed Elshikha\*\*, Georges Abboud, Carolin Arnold\*\*, Laurence Morel, **Sihong Song\*** (2023) *Alpha 1 antitrypsin protects against bm 12 induced lupus transgenic mouse models.* (in preparation).

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42. Lowe, K. C.\*\*, Song, S. (2004) *Construction and In Vitro Evaluation of a Novel AAV Vector for Type I Diabetes Mellitus*. AACP annual meeting, Salt Lake City, UT, July 14, 2004.
43. Witek, R. P., **Song, S.**, Lu, Y.\*\*, Choi, Y-K.\*\*, Zheng, D., Jorgensen, M., Li, C., Flotte, T. R., Petersen, B. E. (2004) *Ex vivo transduced liver stem cells as a platform for gene therapy*. FASEB Summer Research Conferences, Mechanisms of Liver Growth, Development and Disease. Snowmass Village, Colorado, August 7-12, 2004.
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45. Choi, Y-K.\*\*, Lu, Y.\*\*, **Song, S.\*** (2004) *Effect of hepatocyte division on rAAV integration*. Xth Parvovirus Workshop, St. Petersburg, FL, September 11, 2004.
46. Choi, Y-K.\*\*, Lu, Y., Han, Y., Tang, Q., Zhao, G.\*\*, Byrne, B. J., Berns, K. I., Flotte, T. R. **Song, S.\*** (2004) *Host cell effect on the molecular fate of rAAV genome*. Xth Parvovirus Workshop, St. Petersburg, FL, September 11, 2004.
47. **Song, S.\***, Witek, R. P. Lu, Y.\*\*, Choi, Y-K.\*\*, Zheng, D., Jorgensen, M., Li, C., Flotte T. R. Petersen, B. E. (2004) *Transplantation of ex vivo transduced liver*

- progenitor cells by rAAV vector.* Xth Parvovirus Workshop, St. Petersburg, FL, September 11, 2004.
48. Lu, Y.\*\*, Tang, M.\*\*, Wasserfall, C., Choi, Y-K.\*\*, Gardmann, T., Campbell-Thompson, M., Atkinson, M. A., **Song, S.\*** (2004) *Prevention of Type 1 Diabetes by Alpha 1 Antitrypsin Gene Therapy.* ADA annual meeting, June 4-8, Orlando.
  49. Choi, Y-K.\*\*, Barry J. Byrne, B. J., **Song, S.\*** (2004) *Targeting DNA-PK by siRNA to study AAV replication.* ASGT annual meeting, June 2-6, Minneapolis.
  50. Lu, Y.\*\*, Tang, M.\*\*, Wasserfall, C., Choi, Y-K.\*\*, Gardmann, T., Campbell-Thompson, M., Atkinson, M. A., **Song, S.\*** (2004) *Alpha 1 antitrypsin gene delivery for preventing type 1 diabetes in NOD mice.* ASGT annual meeting, June 2-6, Minneapolis..
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  52. **Song, S.\***, Lu, Y.\*, Choi, Y-K.\*\*, Han, Y., Tang, Q., Zhao, G., Berns I.K., and Flotte, T.R., (2004) *DNA Dependent Protein Kinase (DNA-PK) Inhibits Adeno-Associated Virus (AAV) DNA Integration.* Gordon Conference, The Science of Viral Vectors for Gene Therapy, Santa Barbara, CA. Feb 8-13, 2004.
  53. Choi, Y-K.\*\*, Lu, Y.\*\*, **Song, S.\*** (2005) *Effect of hepatocyte division on molecular fate of rAAV DNA.* **Molecular Therapy**, 11(s1):s198. Poster presentation, 8<sup>th</sup> Annual Meeting of ASGT, St. Louis, June 1-5.
  54. Choi, Y-K.\*\*, Zolotukin, I., Byrne, B. and **Song, S.\*** (2005) *Effect of DNA-PKcs rAAV replication.* **Molecular Therapy**, 11(s1):s199. Poster presentation, 8<sup>th</sup> Annual Meeting of ASGT, St. Louis, June 1-5.
  55. Brantly, M. L., Humphries, M., **Song, S.**, Conlon, T., Poirier, A., Byrne B. J., Snyder R., Flotte, T. R. (2005) Phase I clinical trials of recombinant adeno-associated virus (rAAV)-alpha-1 antitrypsin vectors. **Molecular Therapy** 11(s1):s415. Oral presentation, 8th Annual Meeting of ASGT, St. Louis, June 1-5, 2005.
  56. Tang, M.\*\*, Lu, Y.\*\*, Brusko, T., Wasserfall, C., Zhang, B.\*\*, Campbell-Thompson, M., Atkinson, M. and **Song, S.\*** *Prevention of type 1 diabetes by AAT gene therapy is dose and time dependent.* **Diabetes**, 54(s1):A312. Poster presentation, 65<sup>th</sup> ADA annual meeting, San Diego, June 10-14, 2005.
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59. **Song, S.\***, Lu, Y\*\*., Tang, M.\*\*., Zhang, B.\*\*., Wasserfall, C., Campbell-Thompson, M., Atkinson, M. (2006) *Alpha 1 Antitrypsin (AAT) Gene Therapy for the Prevention of Type 1 Diabetes*. **Molecular Therapy**, 13: s30.
60. Glushakova, L.G., Gorbatusky, M., Lu, Y.\*\*., **Song, S.\***, Zheng, M., Shinohara, T., Hauswirth, W.W. (2006) *Transfer of LEDGF to the Mouse Retina Via Systemic AAV Vector Administration*. **Molecular Therapy**, 13: s192.
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63. Zhang, B.\*\*., Compbell-Thompson, M., and **Song, S.\*** (2006) *Alpha 1 antitrypsin gene expression in islet cells*. **Diabetes**, 55 (supplement):A357.
64. Lu, Y., and **Song, S.\*** (2007) *Differential immune responses to transgene products from rAAV1 and rAAV8 vectors*. **Molecular Therapy**, 15: s323.
65. Li, H., Lu, Y., Witek, R., Chang, L-J., Campbell-Thompson, M., Flotte, T., Petersen, B., and **Song, S.\*** (2007) *Ex vivo transduction and transplantation of bone marrow cells for liver gene delivery of alpha 1 antitrypsin (AAT)*. **Molecular Therapy**, 15: s398.
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67. Choi, Y-K.\*\*., Grimstein, C.\*\*., Compbell-Thompson, M., Brantly, M., Spencer, T., and **Song, S.\*** (2007) *Effect of alpha 1 antitrypsin (AAT) on rheumatoid arthritis development in mouse model*. Poster presentation in 6<sup>th</sup> International Scientific Conference New Insights into the Biology of AAT: The Expanded Role of AAT in the Treatment of AAT Deficient Individuals and Other Diseases, Coral Gables, Florida, Feb 8-10, 2007.

68. Zhang, B.\*\* , Lu, Y\*\*., Campbell-Thompson, M., Wasserfall, C., Atkinson, M. Brantly, M. and **Song, S.\*** (2007) *Alpha 1 antitrypsin protects against islet cell apoptosis and prevents diabetes*. Poster presentation in 6<sup>th</sup> International Scientific Conference New Insights into the Biology of AAT: The Expanded Role of AAT in the Treatment of AAT Deficient Individuals and Other Diseases, Coral Gables, Florida, Feb 8-10, 2007.
69. Zhang, B.\*\* , Tang, M.\*\* , Wasserfall, C., Atkinson, M. Brantly, M. and **Song, S.\*** (2007) *Alpha 1 antitrypsin (AAT) inhibits Granzyme B activity and NK cell killing*. Poster presentation in 6<sup>th</sup> International Scientific Conference New Insights into the Biology of AAT: The Expanded Role of AAT in the Treatment of AAT Deficient Individuals and Other Diseases, Coral Gables, Florida, Feb 8-10, 2007.
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71. Zhang, B.\*\* , Tang, M. \*\* , Wasserfall, C., Atkinson, M., **Song, S.\*** (2007) “*Alpha 1 antitrypsin (AAT) inhibits granzymes B activity and NK cell killing*”. **Diabetes**, 56:a59.
72. Lu, Y. \*\* , Zhang, B. \*\* , Choi, YK. \*\* , Campbell-Thompson, M. Wasserfall, C. Brantly, M. Atkinson, M. **Song, S.\*** (2007) *Induction of type 1 hypersensitivity in Nod mice subjected to alpha 1-antitrypsin (AAT) protein therapy*. **Diabetes**, 56:a324.
73. Molano, RD., Pileggi, A., **Song, S.**, Zahr, E., Sanjose, S., Wasserfall, C., Ricordi, C., Atkinson, MA and Inverardi, L. (2007) *The role of humoral immunity in Alpha 1 Antitrypsin-mediated islet allograft survival prolongation*. **Diabetes**, 56:a524.
74. **Song, S.\*** Molano, RD., Pileggi, A., Lu, Y.\*\* , Wasserfall, C., Campbell-Thompson, M., Zahr, E., Sanjose, S., Ricordi, C., Atkinson, MA. and Inverardi, L. (2007) *Islet allograft survival under systemic Alpha 1 Antitrypsin treatment: Comparison of gene therapy versus protein therapy*. **Diabetes**, 56:a525.
75. **Song, S.\***, Pileggi, A., Molano, RD., Lu, Y.\*\* , Campbell-Thompson, M., Wasserfall, C., Zahr, E., Sanjose, S., Ricordi, C., Inverardi, L. and Atkinson, MA. (2007) *Alpha 1 Antitrypsin therapy does not prevent recurrence of autoimmunity in NOD mice*”. **Diabetes**, 56:a525.
76. Pileggi, A., Molano, RD., **Song, S.**, Zahr, E., Sanjose, S., Villate, S., Wasserfall, C., Ricordi, C., Atkinson, MA. and Inverardi, L. (2007) *Effects of alpha-1 Antitrypsin on islet allograft survival in spontaneously diabetic NOD mice*. **Diabetes**, 56:a719.
77. Li, H.\*\* , Lu, Y.\*\* , Zhang, B.\*\* , Petersen, B., **Song, S.\*** (2008) *Adult stem cell mediated liver gene delivery of alpha 1 antitrypsin*. **Molecular Therapy**, 16: s356.
78. Zhang, B.\*\* , Lu, Y.\*\* , **Song, S.\*** (2008) *Adeno-associated viral vector-mediated glucagon-like peptide 1 gene therapy improves glucose metabolism in type 2 diabetic (db/db) mice*. **Molecular Therapy**, 16: s294.

79. Grimstein, C.\*\* , Choi, Y-K.\*\* , Wasserfall, C., Atkinson, M., Campbell-Thompson, M., and **Song, S.\*** (2008) *Alpha 1 antitrypsin (AAT) for rheumatoid arthritis* **International Journal of Rheumatic Diseases** 11(supplement 1):A32.
80. Grimstein, C.\*\* , Choi, Y-K.\*\* , Sato, M., and **Song, S.\*** (2008) *Protein and adeno-associated virus mediated gene therapy of alpha-1 antitrypsin reduces autoantibody production and inhibits arthritis in collagen-induced arthritis*. American Association of Pharmaceutical Scientists (AAPS), 2008 National Biotechnology Conference, Toronto, Canada. June 22 – 25, 2008 (G.C. received AMGEN Travelship award for 2008 AAPS National Biotechnology Conference and Graduate student symposium award at 2008 AAPS National Biotechnology Conference).
81. Grimstein, C.\*\* , Choi, Y-K.\*\* , Wasserfall, C., Satoh, M., Atkinson, M., Campbell-Thompson, M., **Song, S.\*** (2008) *Alpha 1 antitrypsin (AAT) gene therapy in collagen-induced arthritis*. **Molecular Therapy**, 16: s129. Oral presentation at American Society of Gene Therapy (ASGT) 11th Annual Meeting, May 28 – June 1, 2008, Boston, MA. (G.C. received Travel award for 2008 ASGT Annual Meeting)
82. Min SH, Miller R, Choi YK\*\* , **Song S.** Chiodo V, Kuro-O M, Hauswirth WW (2008) *Expression of Klotho and Its Possible Role in a Visual Function of Mouse Retina*. **Investigative Ophthalmology & Visual Science** 49 (13), 784-784
83. Zhang, B.\*\* , Atkinson, M., and **Song, S.** (2009) *Alpha 1-antitrypsin (AAT) enhances the function of pancreatic beta-cells*. **Diabetes**, 58:A416.
84. Le, H. T\*\*., Hirko, A. C., **Song, S.**, Hughes, J. A. (2009) Brain injury in stroke protected by Gelsolin and AAT therapies. Poster presentation in AAPS Annual Meeting and Exposition, November 8-12, 2009, Los Angeles, CA.
85. Ma, H.\*\* , Lu, Y.\*\* , Li, W.\*\* , Wasserfall, C., Haller, M., Campbell-Thompson, M., Brantly, M., Schatz, D., Atkinson, M., and **Song, S.\*** (2009) *Prevention and Reversal of type 1 diabetes by alpha 1 antitrypsin (AAT) in NOD mice*. **Diabetes**, 58:A50.
86. Lu, Y.\*\* , **Song, S.\*** (2010) rAAV infect mouse bone marrow-derived dendritic cells through receptors with  $\alpha 2,3$  and  $\alpha 2,6$  sialic acids. **Molecular Therapy** 18: s146.
87. Ren, Y\*\*., Zhang, L., Meyer, EM., Hughes JA. **Song, S.\*** (2010) Effect of alpha 7 nicotinic receptor gene delivery on processes underlying cell viability and cell differentiation. **Molecular Therapy**, 18: s302
88. Lu, Y.\*\* , **Song, S.\*** (2010) Immune tolerance induced by AAV vectors. Poster presentation, Keystone Symposia, Taos, New Mexico, Feb 21-26, 2010.
89. Le, H., Hirko, A., Legler, C., Thinschmidt, J., King, M., Hughes, J., Song, S. (2010) The Protective Effect of Gelsolin and Alpha 1-Anti trypsin in endothelin-induced middle cerebral artery occlusion in rats. Neuroscience Meeting, Nov. 13-17, 2010, San Diego.
90. Lu, Y. \*\* , Theisen, L. \*\* , Grimstein, C.\*\* , Park, M., Wasserfall, C., Atkinson, M. and **Song, S.\*** (2011) rAAV8 mediated negative vaccine therapy in NOD mouse model. **Molecular Therapy** 19: s265.

91. Fueth, M. \*\*, **Song, S.** \* (2011) *Alpha-1-antitrypsin prevents doxorubicin-induced cardiomyopathy*. Poster presentation in AAPS Annual Meeting and Exposition, Oct 23-27, 2011, Washington, DC.
92. Le, H., Hirko, A., Thinschmidt, J., King, M., Hughes, J., **Song, S.** \* (2011) *The protective effects of alpha-1 antitrypsin in endothelin-induced middle cerebral artery occlusion in rats*. Poster presentation in AAPS Annual Meeting and Exposition, Oct 23-27, 2011, Washington, DC.
93. Wang, RL., Xiao, CK., Lu, Y\*\*, Oshins, RA., McAndrew, EJ, Fu, AD., Liu, C., **Song, S.**, Rouhani, FN., and Brantly, ML. *Expression of the Normal Alpha-1-Antitrypsin Gene in Transgenic PI\*Z Mice is Associated with Decreased Polymerization of the Z Mutant and Increased Secretion of Z and Normal Alpha-1-Antitrypsin*. The 6<sup>th</sup> International Symposium on the Chemistry and Biology of Serpins, Oct. 23-26, 2011, Chapel Hill, NC.
94. Xiao K, Wang RL, Lu Y\*\*, Oshins RA, Bridges RL, McAndrew EJ, Huegel A, Fu AD, Liu C, Song S, Rouhani FN, Brantly ML, *Overexpression of human alpha-1 antitrypsin (AAT) in PiZZ liver reduce the polymerization and facilitate secretion in vitro and in vivo*, (American Society of Cell biology Annual Meeting), Mol. Biol. Cell 22, Abstract No. 2358, Dec. 2011.
95. Mong-Jen Chen\*\*, Yuanqing Lu\*\*, Takashi Hamazaki, Hsin-Yin Tsai, Kirsten Erger, Thomas Conlon, Arun Srivastava, Mark Brantly, Vince Chiodo, William Huaswirth, Naohiro Terada and **Sihong Song**\*. (2013) *Reprogramming adipose tissue derived mesenchymal stem cells (AT-MS) into pluripotent stem cells by a mutant AAV vector*. **Molecular Therapy** 21: s210.
96. Maria Aparecida B. L. Seabra, Ahmed S. Elshikha\*\*, Andrea Ritter\*\*, Osnir S.Viana, **Sihong Song**, Adriana Fontes, Guenther Hochhaus, Beate S. Santos *Toxicity Study of Aqueous CdTe Nanocrystals using U87 and GL 261 cells lines* Poster presentation (M103) in The 8th International Conference on Quantum Dots, Pisa (May 11th-16th 2014).
97. Mohammad Akbar\*\*, Ahmed Elshikha\*\*, Mong-Jen Chen\*\*, Wael Hegazy\*\*, Lexie Holliday, **Sihong Song**\* (2014) *Therapeutic Potential of Alpha-1 Antitrypsin and Mesenchymal Stem Cells for the Treatment of Osteoporosis*. Poster Presentation (T3001) at the 2014 AAPS Annual Meeting and Exposition, November 2-6, 2014, San Diego, CA.
98. Mohammad Akbar\*\*, Yuanqing Lu\*\*, Mong-Jen Chen\*\*, Ahmed Elshikha\*\*, Rubina Ahmed \*\*, Mark Brantly, Long-ji Chang, Lexie Holliday, Jay J. Cao, **Sihong Song**\* (2015) *Alpha 1 antitrypsin (AAT) gene and stem cell based therapies for the treatment of osteoporosis*. Oral presentation at American Society of Gene Therapy (ASGT) 18th Annual Meeting, May 13–16, 2015, New Orleans, LA. **Molecular therapy**, 23(S1):S203. **M.A. received Meritorious Abstract Travel Award**

99. Yuanqing Lu\*\*, Ahmed Elshikha\*\*, George, W. Marek, Mohammad Akbar\*\*, Guohua An, Mark Brantly, **Sihong Song** (2015) Bortezomib enhances AAV vector mediated transduction, but inhibited the secretion of transgene product. Poster presentation at American Society of Gene Therapy (ASGT) 18th Annual Meeting, May 13–16, 2015, New Orleans, LA. *Molecular therapy*, 23(S1):S41
99. Mohammad Ahsanul Akbar\*\*, Yuanqing Lu\*\*, Ahmed S. Elshikha\*\*, Rubina Ahamed\*\*, Mark Brantly, L. Shannon Holliday, Jay J. Cao and **Sihong Song**\*. Alpha-1 Antitrypsin (AAT) Gene Delivery by Recombinant Adeno Associated Virus Vector for the Treatment of Osteoporosis. Accepted abstract number NBC-15-356, 2015 AAPS National Biotechnology Conference in San Francisco, California, June 8-10, 2015. **This abstract also accepted for AAPS Annual Meeting, October 26-30, 2015 as an anchor abstract. This abstract was also presented in the AABPS (American Association of Bangladeshi Pharmaceutical Scientist) 3rd convention, August 8-10, 2015 as an anchor abstract.**  
**Note: MAA received Amgen travel award from AAPS**
100. Ye Yuan\*\*, Ying Li, Denis Titov, Brian Brenner, Lee Seifer, Nurdina Karic, Yuanqing Lu\*\*, **Sihong Song**\*, Lei Zhou, Anti-aging Effect of Human Alpha 1 Antitrypsin (hAAT) in Drosophila Model. Poster Presentation at 2015 Graduate Student Research Day, University of Florida Institute on Aging, University of Florida, Gainesville, FL, September 30, 2015.
101. Mohammad Ahsanul Akbar\*\*, Yuanqing Lu\*\*, Ahmed S. Elshikha\*\*, Rubina Ahamed\*\*, Mark Brantly, L. Shannon Holliday, Jay J. Cao and **Sihong Song**\*. Alpha-1 antitrypsin (AAT) Gene Delivery by Recombinant Adeno Associated Virus Vector for the Treatment of Osteoporosis. American Society of Bone and Mineral Research, Seattle, October 12, 2015. *J Bone Miner Res* 30 (Suppl 1).
102. Ye Yuan\*\*, Ying Li, Denis Titov, Brian Brenner, Lee Seifer, Nurdina Karic, Yuanqing Lu\*\*, **Sihong Song**\*, Lei Zhou, Anti-aging Effect of Human Alpha 1 Antitrypsin (hAAT) in Drosophila Model. Poster Presentation at 6th Annual Spotlight on Aging Research, University of Florida, Gainesville, FL, October 27, 2015
103. Ahmed S. Elshikha\*\*, Yuanqing Lu\*\*, Mong-Jen Chen\*\*, Andrea Ritter\*\*, Leilani Zeumer, Mohamed Akbar, Laurence M. Morel and **Sihong Song**\*. “Alpha 1 antitrypsin inhibits dendritic cell differentiation and attenuates nephritis in a mouse model of lupus”. Poster presentation at AAPS Annual Meeting and Exposition- Oct. 25–29, Orange County Convention Center, Orlando, Florida, **2015. ASE received UF GSC Travel Grant award**
104. Ahmed S. Elshikha\*\*, Yuanqing Lu\*\*, Mong-Jen Chen\*\*, Andrea Ritter\*\*, Ye Yuan\*\*, Leilani Zeumer, Mohamed Akbar, Laurence M. Morel and **Sihong Song**\*. “A novel therapy for the treatment of lupus in animal models”. Poster presentation at 29<sup>th</sup> annual research showcases and awards recognition day, January 22, **2016.**
105. Ye Yuan\*\*, Ying Li, Denis Titov, Brian Brenner, Lee Seifer, Nurdina Karic, Yuanqing Lu\*\*, **Sihong Song**\*, Lei Zhou, Anti-inflammaging effect of human alpha 1

- antitrypsin. Poster Presentation at 29<sup>th</sup> Annual Research Showcase, University of Florida College of Pharmacy, University of Florida, Gainesville, FL, January 22, 2016
106. Mohammad Ahsanul Akbar\*\*, Yuanqing Lu\*\*, Mong-Jen Chen\*\*, Ahmed S. Elshikha\*\*, Rubina Ahamed\*\*, Mark Brantly, Lung-ji Chang, L. Shannon Holliday, Jay J. Cao and **Sihong Song\***. Alpha-1 Antitrypsin (AAT) gene and stem cell based therapies for the treatment of osteoporosis. Oral Presentation, College of Pharmacy Annual Research Showcase January 22, 2016. **MAA received plaque as a finalist**
  107. Ahmed S. Elshikha\*\*, Yuanqing Lu\*\*, Mong-Jen Chen\*\*, Andrea Ritter\*\*, Ye Yuan\*\*, Leilani Zeumer, Mohamed Akbar, Laurence M. Morel and **Sihong Song\***. “Alpha 1 Antitrypsin Protein & Gene Therapies for the Treatment of Lupus in Animal Models”. Oral presentation at ASGCT 19th Annual meeting, Washington, DC, May 4-7, 2016. *Molecular therapy*, 24(S1):S37. **ASE received Meritorious Abstract Travel Award**
  108. Mohammad Ahsanul Akbar\*\*, Jay J. Cao Yuanqing Lu\*\*, David Nardo, Mong-Jen Chen\*\*, Ahmed S. Elshikha\*\*, Rubina Ahamed\*\*, Mark Brantly, L. Shannon Holliday, and **Sihong Song\***. Alpha-1 Antitrypsin Gene Therapy Prevented Bone Loss in an Ovariectomy Induced Osteoporosis Mouse Model. ASGCT annual conference, Washington DC, May 4-7, 2016. *Molecular therapy*, 24(S1):S250
  109. Ye Yuan\*\*, Ying Li, Denis Titov, Brian Brenner, Lee Seifer, Nurdina Karic, Yuanqing Lu\*\*, **Sihong Song\***, Lei Zhou, Inhibition of Inflammaging by Human Alpha 1 Antitrypsin. Poster Presentation at 2016 AAPS National Biotechnology Conference, Boston, MA, May 13-17, 2016.
  110. Ye Yuan\*\*, Ying Li, Denis Titov, Brian Brenner, Lee Seifer, Nurdina Karic, Mohammad Akbar\*\*, Lei Zhou, **Sihong Song\***,. Anti-inflammaging effect of human alpha 1 antitrypsin. Poster Presentation at Immunology 2016, Seattle, WA, May 13-17, 2016. *J Immunol* 2016 196:54.13.  
[https://www.jimmunol.org/content/196/1\\_Supplement/54.13](https://www.jimmunol.org/content/196/1_Supplement/54.13)
  111. Ye Yuan \*\*, Ying Li, Benedetto DiCiaccio, Denis Titov, Brian Brenner, Lee Seifer, Ahmed Elshika\*\*, Mohammad Akbar \*\*, **Sihong Song\***, Lei Zhou. *Anti-inflammaging Effect of Human Alpha 1 Antitrypsin*. Poster presentation in International Society on Aging and Disease (ISOAD), Sept. 30-Oct. 1, 2016, Stanford University, California.
  112. Mohammad Ahsanul Akbar\*\* and **Sihong Song\*** *Alpha-1 Antitrypsin Inhibits Cathepsin K and Osteoclastic Bone Mineral Resorption In Vitro*. Poster presentation in AAPS Annual Meeting, November 13-17, 2016, Denver.
  113. Ahmed Elshikha\*\*, Yuanqing Lu, Ye Yuan\*\*, Mong-Jen Chen, Mohammad Akbar\*\*, Leilani Zeumer, H. Plate, Hedwig Wolny, Tanja Hoffmann, Johua Allman, Laurence Morel, Sihong Song. *Alpha 1 Antitrypsin Ameliorates Lupus Development in Mouse Models*. Oral Presentation, College of Pharmacy 30<sup>th</sup> Annual Research Showcase February 17, 2017. **AE received plaque as a finalist.**
  114. Ye Yuan\*\*, Benedetto DiCiaccio, Ying Li, Ahmed Elshikha\*\*, Mohammad Akbar\*\*, Yuanqing Lu, Lei Zhou, **Sihong Song\***. *Alpha 1 Antitrypsin, a Promising Drug against Aging and Aging-Related Diseases*. Oral Presentation, College of Pharmacy

- 31<sup>th</sup> Annual Research Showcase February 12, 2018. **YY received plaque as a finalist.**
115. Ahmed Elshikha\*\*, Ye Yuan\*\*, Yuanqing Lu, Mong-Jen Chen, Mohammad Akbar, Hedwig Plate\*\*, Laurence Morel, **Sihong Song\*** *Alpha 1 Antitrypsin Extends Life Span and Ameliorates Lupus Development in Mouse Model*. Poster Presentation, College of Pharmacy 31<sup>th</sup> Annual Research Showcase February 12, 2018.
  116. Ahmed Elshikha\*\*, Ye Yuan\*\*, Yuanqing Lu\*\*, Mong-jen Chen\*\*, Mohammad Akbar\*\*, Hedwig Plate\*\*, Laurence Morel, and **Sihong Song\*** *Targeting Dendritic cells with Alpha 1 Antitrypsin has Therapeutic Potentials in Lupus*. **J Immunol** May 2018, 200 (1 Supplement) 175.3; [https://www.jimmunol.org/content/200/1\\_Supplement/175.3](https://www.jimmunol.org/content/200/1_Supplement/175.3). Oral presentation at Immunology Annual Meeting, Austin, Texas, May 4-8, 2018. **AE received 2018 AAI-Thermo Fisher Trainee Award.**
  117. A. Elshikha\*\*, G. Abboud\*\*, A. Danos\*\*, M. Chen\*\*, Y. Yuan\*\*, L. Zeumer, L. Morel and **S. Song\***. *Protective Effect of Alpha-1-Antitrypsin in a Mouse Model of Lupus Alveolar Hemorrhage*. Poster presentation at 32<sup>nd</sup> annual research showcases and awards recognition day, February 11, 2019. **AE received Graduate Student Award for Research in Aging, Pregnancy or Chronic Diseases.**
  118. Ye Yuan\*\*, Benedetto DiCiaccio, Lei Zhou, **Sihong Song\***, *Alpha 1 Antitrypsin, a Promising Drug against Aging and Aging-Related Diseases*. Oral Presentation at 2018 4<sup>th</sup> International Conference on Health Medicine and Life Sciences, Xi'an, China, Dec 16, 2018.
  119. Ahmed Samir Elshikha\*\*, Georges Abboud, Abigail Danos, Mong-jen Chen\*\*, Ye Yuan\*\*, Leilani Zeumer, Laurence Morel and **Sihong Song\*** *Alpha-1-Antitrypsin Ameliorates Diffuse Alveolar Hemorrhage in the Pristane Induced Lupus Model*. **J Immunol** May 2019, 202 (1 Supplement) 132.12. [https://www.jimmunol.org/content/202/1\\_Supplement/132.12](https://www.jimmunol.org/content/202/1_Supplement/132.12)
  120. Lauren Liu, Mohammed Gholam, Angelica Morales, Carlos Lugo, Julia Abchee, Niharika Bala, Haley Nisonson, Karen Correa, Hassan Moussa, Ahmed Elshikha\*\*, **Sihong Song\***, and Abdel Alli. "Regulation of the renal epithelial sodium channel in alpha-1 antitrypsin knockout mice" Oral presentation at The 61<sup>st</sup> Scientific Meeting of the Southern Salt, Water, and Kidney Club. Lido Key FL. December 2-4, 2021.
  121. Ahmed Elshikha\*\*, Georges Abboud, Laurence Morel, **S. Song\*** "A novel function of human Alpha 1 antitrypsin for the control of autoimmunity: inhibiting toll like receptors in dendritic cells" 35<sup>th</sup> UF College of Pharmacy Research Showcase, Feb 7-8, 2022 (**AE was the winner in postdoc poster group**).
  122. Ahmed S Elshikha\*\*, Georges Abboud, Laurence Morel, and **Sihong Song\*** "Targeting proteolytic cleavage of Toll-Like receptors by alpha-1 antitrypsin inhibited dendritic cells activation and function" Late Breaking Poster presentation, Immunology 2022, May 6-10, 2022 (**AE received a travel award**).
  123. Jordan Stokes\*\*, Zhihang Shen, Cameron Courtney\*\*, Yuanqing Lu, Chenglong Li

and **Sihong Song\*** “*Overexpression of Computationally designed Human IL-10 Mutants*”, 36th UF College of Pharmacy Research Showcase, Feb 6-7, 2023.

#### **Book Chapters:**

1. **Sihong Song\***, Yuanqing Lu\*\*, Ahmed S. Elshikha\*\* (2018). *Chapter 10: In vivo Analysis of Alpha 1 Antitrypsin Functions in Autoimmune Disease Models. Serpins: Methods and Protocols* ISBN: 978-1-4939-8644-6. Series Title: Methods in Molecular Biology V1826. Page:143-155.
2. **Sihong Song\*** and Yuanqing Lu\*\*. (2018) *Chapter 12: Gene Delivery of Alpha-1-Antitrypsin Using Recombinant Adeno-Associated Virus (rAAV). Serpins: Methods and Protocols* ISBN: 978-1-4939-8644-6. Series Title: Methods in Molecular Biology V1826. Page: 183-196.

#### **Invited or Non-peer reviewed articles:**

1. Song, S. (2004) Book Review of “*Generation of cDNA Libraries. Methods and Protocols*” *Pharmaceutical Research*, 21 (3):545

#### **Thesis and Dissertation:**

1. Sihong Song (1989) *Strategy for developing animal husbandry in Daqing City*. M. S. Thesis, Jilin Agricultural University, Changchun, China.
2. Sihong Song (1996) *Structural and Functional Analysis of the Endometrial Gene Encoding Insulin-like Growth Factor-Binding Protein-2*. Ph.D. Dissertation, University of Florida.