BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors.

NAME: Pemmaraju, Naveen

eRA COMMONS USERNAME: NPEMMARAJU

POSITION TITLE: Associate Professor

EDUCATION/TRAINING:

INSTITUTION AND LOCATION	DEGREE	Completion Date	FIELD OF STUDY
Tulane University, New Orleans, LA	BS, Cum Laude	05/2001	Cell and Molecular Biology
University of Arkansas for Medical Sciences, Little Rock, AR	MD	05/2005	Medicine
Johns Hopkins Hospital, Osler Internal Medicine Program, Baltimore, MD	Clinical Residency	07/2005-06/2008	Internal Medicine
MD Anderson Cancer Center, Houston, TX	Clinical Fellowship	07/2008-06/2011	Hematology and Medical Oncology

A. Personal Statement

After completing my Internal Medicine training at Johns Hopkins, in the Osler Medical Program, I subsequently completed both hematology and oncology training at MD Anderson Cancer Center (MDACC). I was selected as a chief fellow in my second year of training. I obtained ABIM board certification in Internal Medicine, Hematology and Oncology, and continued my career at MDACC as an Assistant Professor in the Department of Leukemia. In 2017, I was promoted to Associate Professor in the Department of Leukemia. I have an active clinical schedule during which I see patients: 2 full days of clinic per week, and 2 months of inpatient service per year. I have fellow teaching responsibilities as a Leukemia hematology/oncology Course Director. My clinical/translational research work has been focused on improving outcomes and developing novel therapies for adolescents, young adults, and older adult patients with Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN), Myeloproliferative Neoplasms (MPN) and Acute Myeloid Leukemia (AML). I have authored/co-authored over 200 publications in the medical literature and have presented our group's work at national/international meetings: ASCO, ASH, EHA, AACR. Under the mentorship of Dr. Hagop Kantarjian and Dr. Marina Konopleva, I have been able to establish our group's first active BPDCN clinic, which now serves as a nationwide referral base, and an active BPDCN research program with infrastructure and support for clinical and translational studies. I am the PI/co-PI of active clinical trials in the Leukemia department, for a translational sample study of patients with BPDCN, and was awarded a grant for identifying prognostic biomarkers in patients with BPDCN. I have developed key collaborative projects in BPDCN, with clinical, hematopathology and dermatopathology teams; both internally and externally.

1. **Pemmaraju N**, Konopleva M, Lane AA. More on Blastic Plasmacytoid Dendritic-Cell Neoplasms. N Engl J Med 380(7):695-6, 2/2019. e-Pub 2/2019. PMID: 30785714.
2. Sukswai N, Aung PP, Yin CC, Li S, Wang W, Wang SA, Ortega V, Lyapichev K, Nagarajan P, Alfattal R, Angelova E, Tang Z, Loghavi S, Kanagal-Shamanna R, Miranda RN, **Pemmaraju N**, Bhalla K, Konopleva M, Medeiros LJ, Khoury JD. Dual Expression of TCF4 and CD123 is Highly Sensitive and Specific for Blastic Plasmacytoid Dendritic Cell Neoplasm. Am J Surg Pathol. e-Pub 6/2019. PMID: 31261288.
3. Aung PP, Sukswai N, Nejati R, Loghavi S, Chen W, Torres-Cabala CA, Yin CC, Konopleva M, Zheng X, Wang J, Tang Z, Medeiros LJ, Prieto VG, **Pemmaraju N**, Khoury JD. PD1/PD-L1 Expression in Blastic Plasmacytoid Dendritic Cell Neoplasm. Cancers (Basel) 11(5), 5/2019. e-Pub 5/2019. PMID: 31109153.
4. **Pemmaraju N**, Lane AA, Sweet KL, Stein AS, Vasu S, Blum W, Rizzieri DA, Wang ES, Duvic M, Sloan JM, Spence S, Shemesh S, Brooks CL, Balser J, Bergstein I, Lancet JE, Kantarjian HM, Konopleva M. Tagraxofusp in Blastic Plasmacytoid Dendritic-Cell Neoplasm. N Engl J Med 380(17):1628-1637, 4/2019. PMID: 31018069.

B. Positions and Honors

Positions and Employment

2011-2017 Assistant Professor, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, TX

2012-present Co-Chair, Leukemia Hematology Grand Rounds, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, TX

2013-present Course Director, Leukemia Rotation, Hematology/Oncology Fellowship Program, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, TX
2017-present Associate Professor, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, TX
2019-present Hematology Consultant, Cancer Network, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, TX
2020-present Director, Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN) Program, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, TX

Other Experience and Professional Memberships

2009-2010 Chief Fellow Housestaff Education, Hematology/Oncology, The University of Texas MD Anderson Cancer Center Fellowship Program, Houston, TX
2010-present Member, Interviewing Committee, MD Anderson Hematology/Oncology Fellowship Program, The University of Texas MD Anderson Cancer Center, Houston, TX
2012-2017 Member, OncoLog Editorial Board, The University of Texas MD Anderson Cancer Center, Houston, TX
2012-present Member, Fellowship Steering Committee, The University of Texas MD Anderson Cancer Center, Houston, TX
2013-2016 Member, Transfusion Committee, The University of Texas MD Anderson Cancer Center, Houston, TX
2013-present Member, Clinical Competence Committee, The University of Texas MD Anderson Cancer Center, Houston, TX
2013-present Member, Pharmacy and Therapeutics Committee, The University of Texas MD Anderson Cancer Center, Houston, TX
2015-present Member, Clinical Research Committee (CRC), The University of Texas MD Anderson Cancer Center, Houston, TX
2020-2023 Member, The American Society of Hematology (ASH) Committee on Communications, Washington, DC

Honors

2000 Outstanding Peer Health Advocate of the Year, Student Crest Leadership Award
2002-2003 Academic Medical Scholarship, Inaugural Class of 1957 UAMS Alumni
2003-2004 George Link Ackerman Academic Medical Scholarship
2004-2005 Academic Scholarship, Arkansas Medical Society Alliance AMA Foundation
2004-2005 Academic Scholarship, Arkansas Blood and Cancer Society
2004-2005 Outstanding Geriatrics Medical Student, Junior Clerkship Award
2005 Elected, Class President and Student Commencement Speaker, University of Arkansas for Medical Sciences
2005 H. Elvin Shuffield, MD Outstanding Medical Leadership Award
2010 Celgene 2010 Future Leaders Award for Clinical Research in Hematology, Celgene
2010 Jesse H. Jones Outstanding Teaching Fellow Award, MD Anderson Institutional Scholarship
2010-2011 The Kimberly Patterson Fellowship in Leukemia Research
2011-present Advocacy Leadership Institute, American Society of Hematology
2011 Merit Award, American Society of Clinical Oncology
2012 M. D. Anderson Apple Award for Excellence in Patient Education, MD Anderson Cancer Center
2013-present ACP Fellowship, FACP, American College of Physicians
2013-2014 ASH CRTI Program, American Society of Hematology
2018-2019 Ranked Top 10% for Exceptional Communication with Patients and Families, The University of Texas MD Anderson Cancer Center
2020 2020 Quantum Leap Award for Improving Young Adult Patient Care, Dan's House of Hope (DHOH)

C. Contribution to Science

1. **Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)**. BPDCN is a rare, but aggressive hematologic malignancy, which can behave clinically and pathologically as an acute leukemia. There is no standard therapy. Median overall survival in adults range from 8-14 months, and outcomes are poor, despite multi-

agent chemotherapy and stem cell transplantation; if/when available. Urgent improvements to disease patho-biology, identification of novel agents, and new therapeutics are needed for this malignancy. I have been working closely with my collaborators, Dr. Marina Konopleva (MDACC) and Andrew Lane (Dana Farber Cancer Institute) on clinical development of novel therapies for patients with BPDCN. Our goals are to increase our understanding of how the genetics of blood cancer may affect disease prognosis and the effectiveness of treatment; as well as, provide preliminary data to inform the development of new, targeted therapies.

a. **Pemmaraju N**, Lane AA, Sweet KL, Stein AS, Vasu S, Blum W, Rizzieri DA, Wang ES, Duvic M, Sloan JM, Spence S, Shemesh S, Brooks CL, Balser J, Bergstein I, Lancet JE, Kantarjian HM, Konopleva M. Tagraxofusp in Blastic Plasmacytoid Dendritic-Cell Neoplasm. N Engl J Med 380(17):1628-1637, 4/2019. PMID: 31018069.
b. Montero J, Stephansky J, Cai T, Griffin GK, Cabal-Hierro L, Togami K, Hogdal LJ, Galinsky I, Morgan EA, Aster JC, Davids MS, LeBoeuf NR, Stone RM, Konopleva M, **Pemmaraju N**, Letai A, Lane AA. Blastic Plasmacytoid Dendritic Cell Neoplasm is Dependent on BCL-2 and Sensitive to Venetoclax. Cancer Discov. e-Pub 12/2016. PMID: 27986708.
c. Beird HC, Khan M, Wang F, Alfayez M, Cai T, Zhao L, Khoury J, Futreal PA, Konopleva M, **Pemmaraju N**. Features of non-activation dendritic state and immune deficiency in blastic plasmacytoid dendritic cell neoplasm (BPDCN). Blood Cancer J 9(12):99, 12/2019. e-Pub 12/2019. PMID: 31811114.

2. **Myeloproliferative Neoplasms (MPN)**. Another avenue of my research focus is the identification of characteristics in subsets of patients with MPN; including, identification of risk factors, and development of novel, targeted therapies, beyond JAK inhibitors. I have worked closely with Dr. Srdan Verstovsek (MDACC) and collaborated with Dr. Alison Moliterno (Johns Hopkins) and Dr. Ruben Mesa (Mayo Clinic, Scottsdale). I recently presented two first-author oral presentations (LCL161 in MF, SL-401 in MF) during an ASH, MPN, Oral Session in December of 2019.
   a. Mughal TI, **Pemmaraju N**, Radich JP, Deininger MW, Kucine N, Kiladjian JJ, Bose P, Gotlib J, Valent P, Chen CC, Barbui T, Rampal R, Verstovsek S, Koschmieder S, Saglio G, Van Etten RA. Emerging translational science discoveries, clonal approaches and treatment trends in chronic myeloproliferative neoplasms. Hematol Oncol. e-Pub 4/2019. PMID: 31013548.
   b. Benton CB, Boddu PC, DiNardo CD, Bose P, Wang F, Assi R, **Pemmaraju N**, Kc D, Pierce S, Patel K, Konopleva M, Ravandi F, Garcia-Manero G, Kadia TM, Cortes J, Kantarjian HM, Andreeff M, Verstovsek S. Janus kinase 2 variants associated with the transformation of myeloproliferative neoplasms into acute myeloid leukemia. Cancer. e-Pub 2/2019. PMID: 30811597.
   c. **Pemmaraju N**, Kantarjian H, Nastoupil L, Dupuis M, Zhou L, Pierce S, Patel KP, Masarova L, Cortes J, Verstovsek S. Characteristics of patients with myeloproliferative neoplasms with lymphoma, with or without JAK inhibitor therapy. Blood. e-Pub 2/2019. PMID: 30796023.

3. **AYA outcomes research**. The identification of, and improved risk stratification for, a group of unique, vulnerable, leukemia patients between pediatric and adult ages (adolescents and young adults, or AYA) has been an important area of research in my early career development. Working primarily with Dr. Jorge Cortes at MDACC, our group's work has identified worse than expected outcomes in some subsets of AYA leukemia patients; in particular, CML AYA patients. This work has led to new research in our field with regards to new directions, therapies and discussion for this group of patients.
   a. **Pemmaraju N**, Kantarjian HM, Cortes JE, Quintas-Cardama A, Pierce SA, Verstovsek S. Incidence and Outcomes of Myeloproliferative Neoplasms (MPN) in Adolescents and Young Adults (AYAs). ASH Annual Meeting 2012, 2012.
   b. **Pemmaraju N**, Kantarjian H, Ravandi F, Nogueras-Gonzalez GM, Huang X, O'Brien S, Wierda W, Garcia-Manero G, Thomas D, Pierce S, Verstovsek S, Borthakur G, Cortes J. Patient Characteristics and Outcomes in Adolescents and Young Adults (AYA) With Acute Myeloid Leukemia (AML). Clin Lymphoma Myeloma Leuk 16(4):213-222.e2, 4/2016. e-Pub 1/2016. PMCID: PMC4811701.
   c. **Pemmaraju N**, Cortes J. Chronic myeloid leukemia in adolescents and young adults: patient characteristics, outcomes and review of the literature. Acta Haematol 132(3-4):298-306, 2014. e-Pub 9/2014. PMID: 25228555.

4. **Leadership in academic social media for hematologic malignancies**. My work in this area has led to very productive and ongoing external collaborations (Michael Thompson, Aurora Medical Center; Ruben Mesa, Mayo Scottsdale; Vikas Gupta, Princess Margaret) analyzing the utility of social media for healthcare stakeholders in the field of blood cancers. I am the founder of, and have registered, 2 separate Twitter disease-specific hashtag communities: #MPNSM and #BPDCN. Additionally, I was involved with a

project analyzing the #AYACSM community, designed for AYA patients and interested stakeholders. Based on this work, I was invited to serve as Section editor for Current Hematology Malignancy Reports for the first ever series on social media papers in blood cancers. As part of this series, our group has now published several seminal articles in the field detailing the initiation, utilization, and metrics of these novel communities. In pursuing this new area of research, I have been able to focus upon, expand, innovate, and author/co-author papers in my established areas of interest: BPDCN, MPN, and AYA research.

a. **Pemmaraju N**, Gupta V, Mesa R, Thompson MA. Social Media and Myeloproliferative Neoplasms (MPN)-Focus on Twitter and the Development of a Disease-specific Community: #MPNSM. Curr Hematol Malig Rep 10(4):413-20, 12/2015. e-Pub 9/2015. PMID: 26411990.
b. **Pemmaraju N**, Gupta V, Thompson MA, Lane AA. Social Media and Internet Resources for Patients with Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN). Curr Hematol Malig Rep. e-Pub 8/2016. PMID: 27492117.
c. **Pemmaraju N**, Utengen A, Gupta V, Kiladjian JJ, Mesa R, Thompson MA. Social Media and Myeloproliferative Neoplasms (MPN): Analysis of Advanced Metrics from the First Year of a New Twitter Community: #MPNSM. Curr Hematol Malig Rep. e-Pub 8/2016. PMID: 27492118.

Complete List of Published Work in My Bibliography:
http://www.ncbi.nlm.nih.gov/pubmed/?term=pemmaraju+n

D. Additional Information: Research Support and/or Scholastic Performance

Ongoing Research Support

Pemmaraju (PI) 5/14/2014-present
Stemline Therapeutics, Inc.
SL-401 in Patients with Acute Myeloid Leukemia or Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)
To study safety and efficacy of SL-401 in AML or BPDCN.
Role: Principal Investigator

Pemmaraju (PI) 10/1/2014-present
Novartis
Open Label Phase 2 Single agent study of LCL-161 in Patients with Primary Myelofibrosis (PMF), Post-Polycythemia Vera Myelofibrosis (Post-PV MF), or Post-Essential Thrombocytosis Myelofibrosis (Post-ET MF)
To determine the efficacy of LCL161 as therapy for PMF, post-PV MF and post-ET MF.
Role: Principal Investigator

Pemmaraju (PI) 3/26/2015-present
Stemline Therapeutics, Inc.
SL-401 in Patients with Advanced, High Risk Myeloproliferative Neoplasms
To characterize expression of IL-3R/CD123 (and other potentially relevant stem cell and disease markers) on myeloid malignant cells and associated cell populations in the BM (when feasible).
Role: Principal Investigator

Pemmaraju (PI) 8/1/2016-present
Affymetrix
Affymetrix Tumor Profiling Grant Winner: Identifying Prognostic Markers of Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)
To test a total of 30 bone marrow samples from IRB-approved patients with BPDCN that have available clinical data including demographics, treatment history, and additional details regarding disease diagnosis, progression and survival.
Role: Principal Investigator

Pemmaraju (PI) 10/13/2017-10/12/2020
AbbVie
Phase 2 Single-Arm, Open-Label Study Evaluating Tolerability and Efficacy of Navitoclax in Combination with Ruxolitinib in Subjects with Myelofibrosis
To evaluate the effect of the addition of navitoclax to ruxolitinib on spleen volume.
Role: Principal Investigator

Pemmaraju (PI) 11/20/2017-11/19/2020

Dana Farber Partners Cancer Care, Inc.
Phase 1 Study of SL-401 in Combination with Azacitidine in Relapsed/Refractory Acute Myeloid Leukemia (AML) or in Treatment-Naive Subjects with AML Not Eligible for Standard Induction Therapy or in Subjects with High-Risk Myelodysplastic Syndrome (MDS)
To determine the maximum tolerated dose (MTD) or recommended phase 2 dose (RP2D) of SL-401 in combination with azacitidine in this patient population and evaluate the safety of this regimen.
Role: Principal Investigator

Pemmaraju (PI) 1/8/2018-1/7/2021
Cellectis
Phase I, open label dose-escalation study to evaluate the safety, expansion, persistence and clinical activity of a single dose of UCART123 (allogeneic engineered T-cells expressing anti-CD123 chimeric antigen receptor), administered in patients with BPDCN
To assess the safety and tolerability of UCART123 administered to patients with relapsed/refractory and newly diagnosed high-risk AML, and to determine the Maximum Tolerated Dose (MTD) of UCART123.
Role: Principal Investigator

Pemmaraju (PI) 1/31/2019-1/30/2021
Plexxikon
A Phase 1b Dose-escalation Study to Assess the Safety, Pharmacokinetics, Pharmacodynamics, and Preliminary Efficacy of PLX2853 in Subjects with Relapsed or Refractory Acute Myeloid Leukemia or High-risk Myelodysplastic Syndrome
To evaluate the safety and PK of orally administered PLX2853 as a single agent in subjects with relapsed or refractory AML or high-risk MDS and to establish the maximum tolerated dose/recommended Phase 2 dose (MTD/RP2D).
Role: Principal Investigator

Pemmaraju (PI) 2/1/2019-2/1/2024
Daiichi-Sankyo
A Phase I Study of PLX51107 (A Novel Bromodomain Inhibitor) in Combination with Azacytidine for Patients with Myelodysplastic Syndrome and Acute Myeloid Leukemia (AML)
To determine the safety and maximum tolerated dose (MTD) of the combination of PLX51107 and azacytidine (AZA).
Role: Principal Investigator

Pemmaraju (PI) 3/5/2019-3/4/2026
Dana Farber Partners Cancer Care, Inc.
Phase 1 Study of Venetoclax, a BCL2 Antagonist, for Patients with Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)
To determine the maximum tolerated dose (MTD) or recommended phase 2 dose (RP2D) of venetoclax and evaluate the safety of this regimen in BPDCN.
Role: Principal Investigator

GRANT12934011 Konopleva (PI) 9/30/2020-9/29/2024
US Department of Defense (DOD)
Identifying Novel Therapeutic Targets and Combination Strategies for Patients with BPDCN
To understand and overcome resistance to single agent tagraxofusp in BPDCN. Determine the efficacy of combined BCL-2 inhibition, hypomethylating agents, and anti-CD123 therapy in BPDCN.
Role: Co-Investigator

Completed Research Support

Nejati (PI) 2/26/2016-2/25/2017
MD Anderson Division of Pathology and Lab Medicine
Integrative Genomic Profiling of Blastic Plasmacytoid Dendritic Cell Neoplasms
To perform genomic analysis of blastic plasmacytoid dendritic cell neoplasms.
Role: Clinical Collaborator