Stanford

Anupama Narla

Assistant Professor of Pediatrics (Hematology/Oncology)

Pediatrics - Hematology & Oncology

CLINICAL OFFICES

- **Pediatric Hematology and Oncology**

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Bio

CLINICAL FOCUS

- Pediatric Hematology-Oncology

ACADEMIC APPOINTMENTS

- Assistant Professor - University Medical Line, Pediatrics - Hematology & Oncology
- Member, Bio-X
- Member, Maternal & Child Health Research Institute (MCHRI)
- Member, Stanford Cancer Institute

ADMINISTRATIVE APPOINTMENTS

- Chief Fellow, Children's Hospital Boston, (2008-2009)
- Director of Hematology Education, Children's Hospital Boston, (2010-2012)
- Director of Fellow Education Series, Children's Hospital Boston, (2011-2012)

BOARDS, ADVISORY COMMITTEES, PROFESSIONAL ORGANIZATIONS

- Member, American Society of Pediatric Hematology/Oncology (2006 - present)
- Member, American Society of Hematology (2006 - present)
- Member, North American Pediatric Aplastic Anemia Consortium (2013 - present)
- Scientific Committee Member, American Society of Hematology (2013 - present)
- Elected Member, Society of Pediatric Research (2014 - present)
- Liaison, ASH Foundation and Development Committee, American Society of Hematology (2016 - present)

PROFESSIONAL EDUCATION

- Board Certification: Pediatrics, American Board of Pediatrics (2006)
- Board Certification: Pediatric Hematology-Oncology, American Board of Pediatrics (2011)

- Fellowship: Children's Hospital Boston (2009) MA
- Residency: UCSF-Graduate Medical Education (2006) CA
- Internship: UCSF-Graduate Medical Education (2004) CA
- Medical Education: University of Pennsylvania Registrar's Office (2003) PA

Research & Scholarship

CURRENT RESEARCH AND SCHOLARLY INTERESTS

More than a decade ago, researchers discovered that a rare congenital bone marrow failure syndrome, Diamond Blackfan anemia (DBA), is caused by mutations in a ribosomal protein RPS19. Subsequently, my mentor Dr. Benjamin Ebert identified RPS14 as the gene responsible for the profound macrocytic anemia in the 5q- syndrome, a subtype of myelodysplastic syndrome. This reinforced the connection between ribosomal abnormalities and defects in erythropoiesis. Moreover, mutations in other genes required for normal ribosome biogenesis have been implicated in other rare congenital syndromes including Schwachman-Diamond syndrome, X-linked dyskeratosis congenita, Cartilage Hair Hypoplasia and Treacher Collins syndrome. Each of these disorders is associated with specific defects in ribosome biogenesis, which cause distinct clinical phenotypes, most often involving bone marrow failure, and have become collectively known as ribosomopathies.

I have studied the molecular mechanisms by which ribosomal dysfunction leads to bone marrow failure by further characterizing the signaling pathways that are triggered and the subsequent effects on hematopoiesis. I published work on ribosomal haploinsufficiency causing selective activation of p53 in human erythroid progenitor cells and on the effects of a microRNA cooperating in the pathogenesis of the 5q- syndrome. I will continue to focus on understanding the effects of specific drugs on these disorders which may uncover further clues about pathophysiology and as importantly, will directly benefit patients. I have published work on the effects of dexamethasone and lenalidomide, the first line therapies for DBA and 5q- MDS respectively, on erythropoiesis and am an author on a manuscript examining the effects of leucine, a stimulator of the mTOR pathway, in these disorders.

The goal of my lab is to make meaningful contributions to the elucidation of the pathophysiology of ribosomopathies, the development of novel therapies and the care of patients in the field.

CLINICAL TRIALS

- A Study of AG-348 in Adult Participants With Pyruvate Kinase (PK) Deficiency, Recruiting
- Pilot Phase I/II Study of Amino Acid Leucine in Treatment of Patients With Transfusion-Dependent Diamond Blackfan Anemia, Recruiting

Publications

PUBLICATIONS

- **Diagnostic work-up for severe aplastic anemia in children: Consensus of the North American Pediatric Aplastic Anemia Consortium.** *American journal of hematology*
  Shimano, K. A., Narla, A., Rose, M. J., Gloude, N. J., Allen, S. W., Bergstrom, K., Broglie, L., Carella, B. A., Castillo, P., de Jong, J. L., Dror, Y., Geddis, A. E., Huang, et al
  2021
- **The active component of Ginseng, Ginsenoside Rb1, improves erythropoiesis in models of Diamond Blackfan Anemia by targeting Nemo-like Kinase.** *The Journal of biological chemistry*
  Wilkes, M. C., Jung, K., Lee, B. E., Saxena, M., Sathianathen, R. S., Mercado, J. D., Perez, C., Flygare, J., Narla, A., Glader, B., Sakamoto, K. M.
  2021: 100988
- **Comprehensive phenotyping of erythropoiesis in human bone marrow: Evaluation of normal and ineffective erythropoiesis.** *American journal of hematology*
  Yan, H., Ali, A., Blanc, L., Narla, A., Lane, J. M., Gao, E., Papoin, J., Hale, J., Hillyer, C. D., Taylor, N., Gallagher, P. G., Raza, A., Kinet, et al
  2021

- **Disseminated Coccidioidomycosis in Bone Marrow.** *American journal of hematology*
  Narla, A., Narla, J.
  2021

- **Whole exome sequencing of a breast tumor in a patient with Diamond Blackfan anemia.** *Blood cells, molecules & diseases*
  Narla, A. n., Ruddy, K. J., Ebert, B. L., Mar, B. n.
  2021; 89: 102566

- **Staying hydrated is important also for erythroblasts.** *Haematologica*
  Narla, A., Mohandas, N.
  2020; 105 (3): 528–29

- **L-leucine improves anemia and growth in patients with transfusion-dependent Diamond-Blackfan anemia: Results from a multicenter pilot phase I/II study from the Diamond-Blackfan Anemia Registry.** *Pediatric blood & cancer*
  Vlachos, A. n., Atsidaftos, E. n., Lababidi, M. L., Muir, E. n., Rogers, Z. R., Alhushki, W. n., Bernstein, J. n., Glader, B. n., Gruner, B. n., Hartung, H. n., Knoll, C. n., Loew, T. n., Nalepa, et al
  2020: e28748

- **Steroid resistance in Diamond Blackfan anemia associates with p57Kip2 dysregulation in erythroid progenitors.** *The Journal of clinical investigation*
  Ashley, R. J., Yan, H. n., Wang, N. n., Hale, J. n., Dulmovits, B. M., Papoin, J. n., Olive, M. E., Udeshi, N. D., Carr, S. A., Vlachos, A. n., Lipton, J. M., Da Costa, L. n., Hillyer, et al
  2020

- **Enasidenib drives human erythroid differentiation independently of isocitrate dehydrogenase 2.** *The Journal of clinical investigation*
  Dutta, R. n., Zhang, T. Y., Köhnke, T. n., Thomas, D. n., Linde, M. n., Gars, E. n., Stafford, M. n., Kaur, S. n., Nakauchi, Y. n., Yin, R. n., Azizi, A. n., Narla, A. n., Majeti, et al
  2020

- **MMP9 inhibition increases erythropoiesis in RPS14-deficient del(5q) MDS models through suppression of TGF-beta pathways.** *Blood advances*
  Youn, M., Huang, H., Chen, C., Kam, S., Wilkes, M. C., Chae, H., Sridhar, K. J., Greenberg, P. L., Glader, B., Narla, A., Lin, S., Sakamoto, K. M.
  2019; 3 (18): 2751–63

- **INHIBITION OF NEMO-LIKE KINASE IMPROVES ERYTHROPOIESIS IN MODELS OF DIAMOND BLACKFAN ANEMIA**
  Takasaki, K., Wilkes, M., Chen, J., Siva, K., Varetti, G., Dever, D., Youn, M., Chae, H., Mercado, J., Saxena, M., Narla, A., Glader, B., Porteus, et al
  WILEY.2019

- **A case series of pediatric patients with direct antiglobulin test negative autoimmune hemolytic anemia.** *Transfusion*
  Miller, J., Cai, W., Andrews, J., Narla, A.
  2019

- **A fork in the road.** *Blood*
  Narla, A. n., Mohandas, N. n.
  2019; 134 (18): 1484–85

- **The Genetic Landscape of Diamond-Blackfan Anemia** *AMERICAN JOURNAL OF HUMAN GENETICS*
  Ulirsch, J. C., Verboon, J. M., Kazerounian, S., Guo, M. H., Yuan, D., Ludwig, L. S., Handsaker, R. E., Abdulhay, N. J., Fiorini, C., Genovese, G., Lim, E. T., Cheng, A., Cummings, et al
  2018; 103 (6): 930–47

- **Dexamethasone Accelerates the Transition of Human BFU-E to CFU-E and Enhances CFU-E Proliferation through Cell Cycle Regulation**
  Ashley, R., Yan, H., Dulmovits, B., Wang, N., Papoin, J., Lipton, J. M., Narla, M., Narla, A., Blanc, L.
  AMER SOC HEMATOLOGY.2018

- **Chromatin Organization By SATB1 Regulates HSP70 Induction in Early Erythropoiesis and Lost in Diamond Blackfan Anemia**
  Wilkes, M. C., Takasaki, K., Youn, M., Chae, H., Narla, A., Sakamoto, K. M.
  AMER SOC HEMATOLOGY.2018

- **MMP9 Inhibition Rescues the Erythroid Defect in RPS14-Deficient Del(5q) MDS Models**
  Youn, M., Huang, H., Chen, C., Kam, S., Wilkes, M. C., Chae, H., Narla, A., Lin, S., Sakamoto, K. M.
  AMER SOC HEMATOLOGY.2018

- **Leucine for the Treatment of Transfusion Dependence in Patients with Diamond Blackfan Anemia**
  Vlachos, A., Atsidaftos, E., Muir, E., Rogers, Z. R., Lababidi, M., Alhushki, W., Farrar, J. E., Glader, B., Gruner, B., Hartung, H., Knoll, C. M., Nalepa, G., Narla, et al
  AMER SOC HEMATOLOGY.2018

- **Pharmacological Inhibition of Nlk (Nemo-like Kinase) Rescues Erythropoietic Defects in Pre-Clinical Models of Diamond Blackfan Anemia**
  Wilkes, M. C., Chen, J., Siva, K., Veretti, G., Dever, D. P., Youn, M., Chae, H., Mercado, J. D., Saxena, M., Narla, A., Glader, B., Porteus, M., Repellin, et al
  AMER SOC HEMATOLOGY.2018

- **The Genetic Landscape of Diamond-Blackfan Anemia.** *American journal of human genetics*
  Ulirsch, J. C., Verboon, J. M., Kazerounian, S., Guo, M. H., Yuan, D., Ludwig, L. S., Handsaker, R. E., Abdulhay, N. J., Fiorini, C., Genovese, G., Lim, E. T., Cheng, A., Cummings, et al
  2018

- **Developmental differences between neonatal and adult human erythropoiesis** *AMERICAN JOURNAL OF HEMATOLOGY*
  Yan, H., Hale, J., Jaffray, J., Li, J., Wang, Y., Huang, Y., An, X., Hillyer, C., Wang, N., Kinet, S., Taylor, N., Mohandas, N., Narla, et al
  2018; 93 (4): 494–503

- **Erythropoiesis: insights into pathophysiology and treatments in 2017** *MOLECULAR MEDICINE*
  Zivot, A., Lipton, J. M., Narla, A., Blanc, L.
  2018; 24

- **An update on the pathogenesis and diagnosis of Diamond-Blackfan anemia.** *F1000Research*
  Da Costa, L. n., Narla, A. n., Mohandas, N. n.
  2018; 7

- **Erythropoiesis: insights into pathophysiology and treatments in 2017.** *Molecular medicine (Cambridge, Mass.)*
  Zivot, A. n., Lipton, J. M., Narla, A. n., Blanc, L. n.
  2018; 24 (1): 11

- **The severe phenotype of Diamond-Blackfan anemia is modulated by heat shock protein 70** *BLOOD ADVANCES*
  Gastou, M., Rio, S., Dussiot, M., Karboul, N., Moniz, H., Leblanc, T., Sevin, M., Gonin, P., Larghero, J., Garrido, C., Narla, A., Mohandas, N., Vainchenker, et al
  2017; 1 (22): 1959–76

- **Loss of FOXM1 promotes erythropoiesis through increased proliferation of erythroid progenitors.** *Haematologica*
  Youn, M., Wang, N., LaVasseur, C., Bibikova, E., Kam, S., Glader, B., Sakamoto, K. M., Narla, A.
  2017

- **Characterization, regulation, and targeting of erythroid progenitors in normal and disordered human erythropoiesis.** *Current opinion in hematology*
  Dulmovits, B. M., Hom, J., Narla, A., Mohandas, N., Blanc, L.
  2017

- **Erythrocyte adenosine deaminase levels are elevated in Diamond Blackfan anemia but not in the 5q-syndrome** *AMERICAN JOURNAL OF HEMATOLOGY*
  Narla, A., Davis, N. L., Lavasseur, C., Wong, C., Glader, B.
  2016; 91 (12): E501–E502

- **CRISPR/Cas9 ß-globin gene targeting in human haematopoietic stem cells.** *Nature*
  Dever, D. P., Bak, R. O., Reinisch, A., Camarena, J., Washington, G., Nicolas, C. E., Pavel-Dinu, M., Saxena, N., Wilkens, A. B., Mantri, S., Uchida, N., Hendel, A., Narla, et al
  2016

- **A novel pathogenic mutation in RPL11 identified in a patient diagnosed with diamond Blackfan anemia as a young adult** *BLOOD CELLS MOLECULES AND DISEASES*
  Narla, A., Yuan, D., Kazerounian, S., LaVasseur, C., Ulirsch, J. C., Narla, J., Glader, B., Sankaran, V. G., Gazda, H.
  2016; 61: 46–47

- **The road not taken?** *Blood*
  Narla, A., Mohandas, N.
  2016; 128 (7): 886-888

- **Coordinate regulation of residual bone marrow function by paracrine trafficking of AML exosomes** *LEUKEMIA*
  Huan, J., Hornick, N. I., Goloviznina, N. A., Kamimae-Lanning, A. N., David, L. L., Wilmarth, P. A., Mori, T., Chevillet, J. R., Narla, A., Roberts, C. T., Loriaux, M. M., Chang, B. H., Kurre, et al
  2015; 29 (12): 2285-2295

- **Biology of the bone marrow microenvironment and myelodysplastic syndromes.** *Molecular genetics and metabolism*
  Rankin, E. B., Narla, A., Park, J. K., Lin, S., Sakamoto, K. M.
  2015; 116 (1-2): 24-28

- **Jekyll and Hyde: the role of heme oxygenase-1 in erythroid biology.** *Haematologica*
  Narla, A. n., Mohandas, N. n.
  2015; 100 (5): 567–68

- **TNF-mediated inflammation represses GATA1 and activates p38 MAP kinase in RPS19-deficient hematopoietic progenitors.** *Blood*
  Bibikova, E., Youn, M., Danilova, N., Ono-Uruga, Y., Konto-Ghiorghi, Y., Ochoa, R., Narla, A., Glader, B., Lin, S., Sakamoto, K. M.
  2014; 124 (25): 3791-3798

- **L-Leucine improves the anaemia in models of Diamond Blackfan anaemia and the 5q-syndrome in a TP53-independent way** *BRITISH JOURNAL OF HAEMATOLOGY*
  Narla, A., Payne, E. M., Abayasekara, N., Hurst, S. N., Raiser, D. M., Look, A. T., Berliner, N., Ebert, B. L., Khanna-Gupta, A.
  2014; 167 (4): 524-528

- **The emerging importance of ribosomal dysfunction in the pathogenesis of hematologic disorders.** *Leukemia & lymphoma*
  Raiser, D. M., Narla, A., Ebert, B. L.
  2014; 55 (3): 491-500

- **Lenalidomide Causes Selective Degradation of IKZF1 and IKZF3 in Multiple Myeloma Cells** *SCIENCE*
  Kroenke, J., Udeshi, N. D., Narla, A., Grauman, P., Hurst, S. N., McConkey, M., Svinkina, T., Heckl, D., Comer, E., Li, X., Ciarlo, C., Hartman, E., Munshi, et al
  2014; 343 (6168): 301-305

- **L-Leucine improves the anaemia in models of Diamond Blackfan anaemia and the 5q- syndrome in a TP53-independent way.** *British journal of haematology*
  Narla, A. n., Payne, E. M., Abayasekara, N. n., Hurst, S. N., Raiser, D. M., Look, A. T., Berliner, N. n., Ebert, B. L., Khanna-Gupta, A. n.
  2014; 167 (4): 524–28

- **Diminutive somatic deletions in the 5q region lead to a phenotype atypical of classical 5q-syndrome** *BLOOD*
  Vlachos, A., Farrar, J. E., Atsidaftos, E., Muir, E., Narla, A., Markello, T. C., Singh, S. A., Landowski, M., Gazda, H. T., Blanc, L., Liu, J. M., Ellis, S. R., Arceci, et al
  2013; 122 (14): 2487-2490

- **Mitochondrial Atpif1 regulates haem synthesis in developing erythroblasts** *NATURE*
  Shah, D. I., Takahashi-makise, N., Cooney, J. D., Li, L., Schultz, I. J., Pierce, E. L., Narla, A., Seguin, A., Hattangadi, S. M., Medlock, A. E., Langer, N. B., Dailey, T. A., Hurst, et al
  2012; 491 (7425): 608-612

- **L-leucine improves the anemia and developmental defects associated with Diamond-Blackfan anemia and del(5q) MDS by activating the mTOR pathway** *BLOOD*
  Payne, E. M., Virgilio, M., Narla, A., Sun, H., Levine, M., Paw, B. H., Berliner, N., Look, A. T., Ebert, B. L., Khanna-Gupta, A.
  2012; 120 (11): 2214-2224

- **Fulminant thrombotic microangiopathy in Pediatrics: Where diagnostic and therapeutic dilemmas meet** *AMERICAN JOURNAL OF HEMATOLOGY*
  Renella, R., Stickney, C., Keswani, M., Mancuso, T., Casavant, D., Ferguson, M., Narla, A.
  2012; 87 (8): 816-818

- **Coordinate loss of a microRNA and protein-coding gene cooperate in the pathogenesis of 5q(-) syndrome** *BLOOD*
  Kumar, M. S., Narla, A., Nonami, A., Mullally, A., Dimitrova, N., Ball, B., McAuley, J. R., Poveromo, L., Kutok, J. L., Galili, N., Raza, A., Attar, E., Gilliland, et al
  2011; 118 (17): 4666-4673

- **Translational medicine: ribosomopathies** *BLOOD*
  Narla, A., Ebert, B. L.

2011; 118 (16): 4300-4301

- **Dexamethasone and lenalidomide have distinct functional effects on erythropoiesis** *BLOOD*
  Narla, A., Dutt, S., McAuley, J. R., Al-Shahrour, F., Hurst, S., McConkey, M., Neuberg, D., Ebert, B. L.
  2011; 118 (8): 2296-2304

- **Neonatal Enteroviral Sepsis/Meningoencephalitis and Hemophagocytic Lymphohistiocytosis: Diagnostic Challenges** *AMERICAN JOURNAL OF PERINATOLOGY*
  Lindamood, K. E., Fleck, P., Narla, A., Vergilio, J., Degar, B. A., Baldwin, M., Wintermark, P.
  2011; 28 (5): 337-345

- **Diamond Blackfan Anemia Treatment: Past, Present, and Future** *SEMINARS IN HEMATOLOGY*
  Narla, A., Vlachos, A., Nathan, D. G.
  2011; 48 (2): 117-123

- **Haploinsufficiency for ribosomal protein genes causes selective activation of p53 in human erythroid progenitor cells** *BLOOD*
  Dutt, S., Narla, A., Lin, K., Mullally, A., Abayasekara, N., Megerdichian, C., Wilson, F. H., Currie, T., Khanna-Gupta, A., Berliner, N., Kutok, J. L., Ebert, B. L.
  2011; 117 (9): 2567-2576

- **Ribosome defects in disorders of erythropoiesis** *INTERNATIONAL JOURNAL OF HEMATOLOGY*
  Narla, A., Hurst, S. N., Ebert, B. L.
  2011; 93 (2): 144-149

- **Difficulty Measuring Methotrexate in a Patient with High-Dose Methotrexate-Induced Nephrotoxicity** *CLINICAL CHEMISTRY*
  Al-Turkmani, M. R., Law, T., Narla, A., Kellogg, M. D.
  2010; 56 (12): 1792-1794

- **Ribosomopathies: human disorders of ribosome dysfunction** *BLOOD*
  Narla, A., Ebert, B. L.
  2010; 115 (16): 3196-3205

- **Allogeneic hematopoietic stem cell transplantation for X-linked ectodermal dysplasia and immunodeficiency: case report and review of outcomes** *IMMUNOLOGIC RESEARCH*
  Permaul, P., Narla, A., Hornick, J. L., Pai, S.
  2009; 44 (1-3): 89-98

- **Blood group antigens in health and disease** *CURRENT OPINION IN HEMATOLOGY*
  Mohandas, N., Narla, A.
  2005; 12 (2): 135-140