Cincinnati Children's

2015 Research Annual Report

Cancer and Blood Diseases Institute

RESEARCH AND TRAINING DETAILS

Click to view members

Faculty	**85**
Joint Appointment Faculty	**26**
Research Fellows	**54**
Research Students	**23**
Support Personnel	**341**
Direct Annual Grant Support	**$16,546,465**
Direct Annual Industry Support	**$1,157,989**
Peer Reviewed Publications	**209**

CLINICAL ACTIVITIES AND TRAINING

Clinical Staff	**11**
Staff Physicians	**10**
Clinical Fellows	**19**
Clinical Students	**4**
Other Students	**8**
Inpatient Encounters	**22,128**
Outpatient Encounters	**26,685**

Division Publications

1. Adams AK, Hallenbeck GE, Casper KA, Patil YJ, Wilson KM, Kimple RJ, Lambert PF, Witte DP, Xiao W, Gillison ML, Wikenheiser-Brokamp KA, Wise-Draper TM, Wells SI. **DEK promotes HPV-positive and -negative head and neck cancer cell proliferation**. *Oncogene*. 2015; 34:868-77.

2. Adams DM, Hammill A. **Other vascular tumors**. *Semin Pediatr Surg*. 2014; 23:173-7.

3. Ahmad N, Adams DM, Wang J, Prakash R, Karim NA. **Hepatic epithelioid hemangioendothelioma in a patient with hemochromatosis**. *J Natl Compr Canc Netw*. 2014; 12:1203-7.

4. Alcamo AM, Pinchasik DE, Mo JQ, Grimley MS, O'Brien MM. **Successful Treatment of Disseminated Adenovirus Infection in an Infant With Acute Lymphoblastic Leukemia**. *J Pediatr Hematol Oncol*. 2014; 37:e178-e181.

5. Aleman MM, Byrnes JR, Wang JG, Tran R, Lam WA, Di Paola J, Mackman N, Degen JL, Flick MJ, Wolberg AS. **Factor XIII activity mediates red blood cell retention in venous thrombi**. *J Clin Invest*. 2014; 124:3590-600.

6. Alferiev IS, Iyer R, Croucher JL, Adamo RF, Zhang K, Mangino JL, Kolla V, Fishbein I, Brodeur GM, Levy RJ, Chorny M. **Nanoparticle-mediated delivery of a rapidly activatable prodrug of SN-38 for neuroblastoma therapy**. *Biomaterials*. 2015; 51:22-9.

7. Andersson C, Kvist PH, McElhinney K, Baylis R, Gram LK, Pelzer H, Lauritzen B, Holm TL, Hogan S, Wu D, Turpin B, Miller W, Palumbo JS. **Factor XIII Transglutaminase Supports the Resolution of Mucosal Damage in Experimental Colitis**. *PLoS One*. 2015; 10:e0128113.

8. Antonini TN, Raj SP, Oberjohn KS, Cassedy A, Makoroff KL, Fouladi M, Wade SL. **A pilot randomized trial of an online parenting skills program for pediatric traumatic brain injury: improvements in parenting and child behavior**. *Behav Ther*. 2014; 45:455-68.

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10. Aydin SE, Kilic SS, Aytekin C, Kumar A, Porras O, Kainulainen L, Kostyuchenko L, Genel F, Kutukculer N, Karaca N, Gonzalez-Granado L, Abbott J, Al-Zahrani D, Rezaei N, Baz Z, Thiel J, Ehl S, Marodi L, Orange JS, Sawalle-Belohradsky J, Keles S, Holland SM, Sanal O, Ayvaz DC, Tezcan I, Al-Mousa H, Alsum Z, Hawwari A, Metin A, Matthes-Martin S, Honig M, Schulz A, Picard C, Barlogis V, Gennery A, Ifversen M, van Montfrans J, Kuijpers T, Bredius R, Duckers G, Al-Herz W, Pai SY, Geha R, Notheis G, Schwarze CP, Tavil B, Azik F, Bienemann K, Grimbacher B, Heinz V, Gaspar HB, Aydin R, Hagl B, Gathmann B, Belohradsky BH, Ochs HD, Chatila T, Renner ED, Su H, Freeman AF, Engelhardt K, Albert MH, inborn errors working party of E. **DOCK8 deficiency: clinical and immunological phenotype and treatment options - a review of 136 patients**. *J Clin Immunol*. 2015; 35:189-98.

11. Aygun B, Mortier NA, Kesler K, Lockhart A, Schultz WH, Cohen AR, Alvarez O, Rogers ZR, Kwiatkowski JL, Miller ST, Sylvestre P, Iyer R, Lane PA, Ware RE, Stroke With Transfusions Changing to Hydroxyurea Trial I. **Therapeutic phlebotomy is safe in children with sickle cell anaemia and can be effective treatment for transfusional iron overload**. *British Journal of Haematology*. 2015; 169:262-6.

12. Ballen KK, Joffe S, Brazauskas R, Wang Z, Aljurf MD, Akpek G, Dandoy C, Frangoul HA, Freytes CO, Khera N, Lazarus HM, LeMaistre CF, Mehta P, Parsons SK, Szwajcer D, Ustun C, Wood WA, Majhail NS. **Hospital length of**

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13. Barnes MJ, Pressey J, Adams J, Hensler MA, Madan-Swain A. **Physician and nurse beliefs of phase 1 trials in pediatric oncology**. *Cancer Nurs*. 2014; 37:E48-52.

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as a novel treatment for gliomas. *Mol Cancer Ther*. 2015; 14:857-64.

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Algorithm Enables Prompt Response to High-Risk Cases of Transplant-Associated Thrombotic Microangiopathy (TMA)

Sonata Jodele, MD

The Cancer and Blood Diseases Institute (CBDI) includes the Divisions of Bone Marrow Transplant and Immune Deficiency, Experimental Hematology, Hematology, and Oncology

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Direct Annual Industry Support	$1.1M
Peer Reviewed Publications	209

Jodele S, Davies SM, Lane A, Khoury J, Dandoy C, Goebel J, Myers K, Grimley M, Bleesing J, El-Bietar J, Wallace G, Chima RS, Paff Z, Laskin BL. Diagnostic and risk criteria for HSCT-associated thrombotic microangiopathy: a study in children and young adults. *Blood.* 2014;124(4):645-653.

PUBLISHED JULY 24, 2014
Blood

After children undergo hematopoietic cell transplantation (HSCT), one of the most severe complications they can develop is thrombotic microangiopathy (TMA). This condition can trigger a cascade of events leading to potentially fatal multi-organ injury.

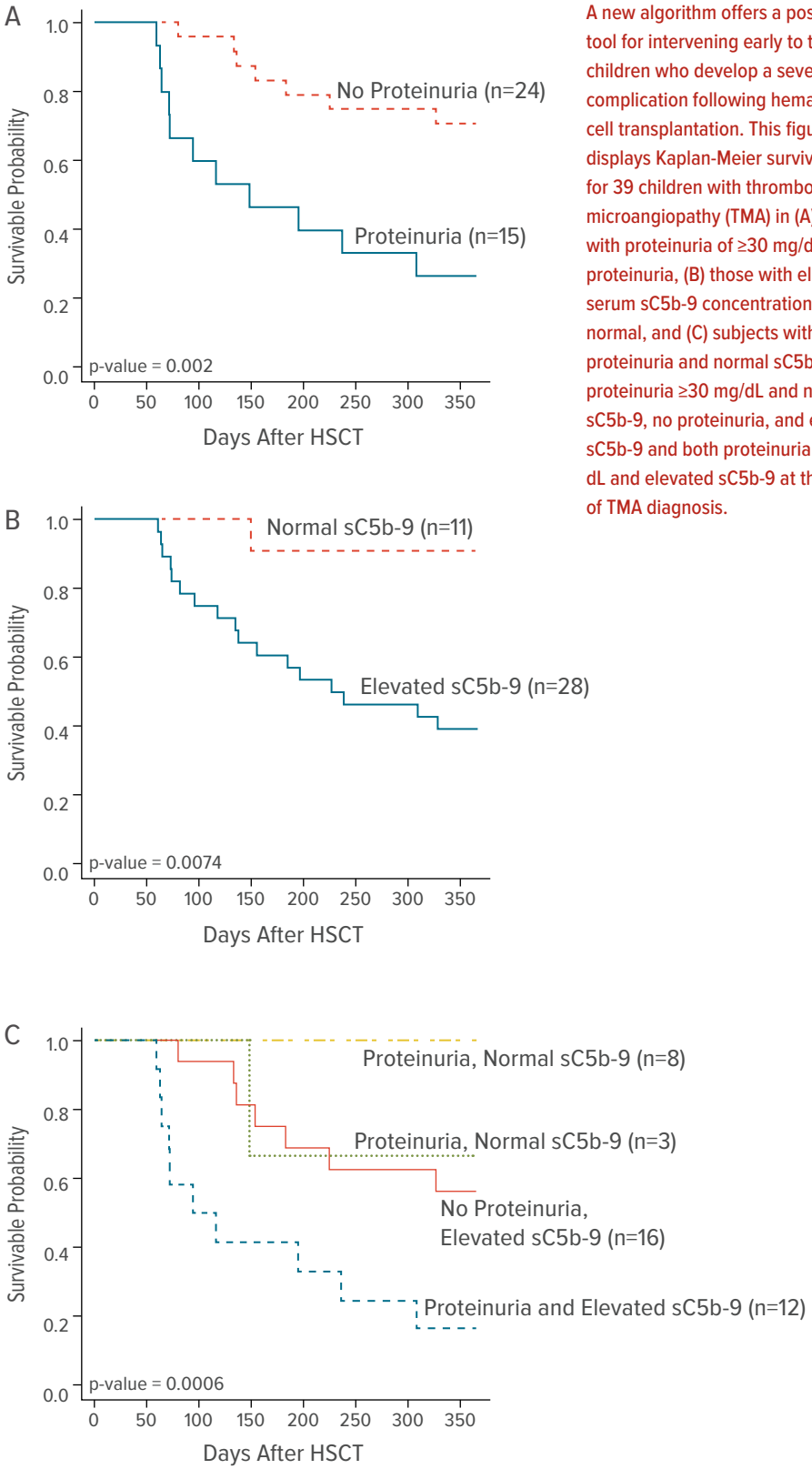
Prompt clinical intervention can save lives, but only if TMA is detected in its earliest stages. In an important paper published July 24, 2014, in the journal *Blood*, a research team led by Sonata Jodele, MD, Division of Bone Marrow Transplantation and Immune Deficiency, reports developing an algorithm that can provide the information clinicians need to act.

The researchers prospectively evaluated 100 HSCT recipients to track TMA incidence and outcomes. They found 39 children who met criteria for TMA. These children had a 43.6 percent non-relapse mortality rate at one year post-transplant, compared to 7.8 percent mortality among children who did not develop TMA.

The team observed that those who died after TMA diagnosis had a greater degree of anemia, higher risk of proteinuria, and were more likely to have evidence of terminal complement activation. Elevated levels of sC5b-9 were present in nearly all subjects with TMA who died but in only about half of those who survived. In contrast, kidney dysfunction assessed by serum creatinine was a very late marker of TMA.

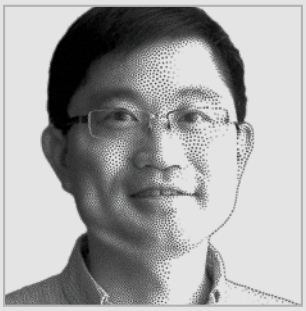
The paper details a series of daily, twice weekly and weekly tests that can detect early TMA markers. Specifically, proteinuria >30 mg/dL as measured by routine dipstick and hypertension >95th percentile were the earliest signs of TMA, along with elevated lactate dehydrogenase (LDH).

These data suggest that complement activation plays a significant role in the pathogenesis of severe TMA after HSCT. The team recommends that patients with proteinuria and evidence of complement activation should be considered for treatment with eculizumab, a humanized monoclonal antibody that functions as a terminal complement inhibitor.



A new algorithm offers a possible tool for intervening early to treat children who develop a severe complication following hematopoietic cell transplantation. This figure displays Kaplan-Meier survival curves for 39 children with thrombotic microangiopathy (TMA) in (A) those with proteinuria of ≥30 mg/dL vs. no proteinuria, (B) those with elevated serum sC5b-9 concentration vs. normal, and (C) subjects with no proteinuria and normal sC5b-9, proteinuria ≥30 mg/dL and normal sC5b-9, no proteinuria, and elevated sC5b-9 and both proteinuria ≥30 mg/dL and elevated sC5b-9 at the time of TMA diagnosis.

Antidepressant Identified as Potential Brain Tumor Suppressor



Qing Richard Lu, PhD

CANCER AND BLOOD DISEASES INSTITUTE RESEARCH AND TRAINING DETAILS

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He X, Zhang L, Chen Y, Remke M, Shih D, Lu F, Wang H, Deng Y, Yu Y, Xia Y, Wu X, Ramaswamy V, Hu T, Wang F, Zhou W, Burns DK, Kim SH, Kool M, Pfister SM, Weinstein LS, Pomeroy SL, Gilbertson RJ, Rubin JB, Hou Y, Wechsler-Reya R, Taylor MD, Lu QR. The G protein alpha subunit Galphas is a tumor suppressor in Sonic hedgehog-driven medulloblastoma. *Nat Med*. 2014;20(9):1035-1042.

PUBLISHED SEPTEMBER 2014
Nature Medicine

An international research team, led by Qing "Richard" Lu, PhD, scientific director of the Brain Tumor Center at Cincinnati Children's, has discovered a novel tumor suppressor gene that could help overcoming rapid drug resistance when treating pediatric brain cancer.

The latest findings specifically address aggressive sonic hedgehog (SHH)-driven medulloblastomas. However, the work may have wider impact. The team showed that Rolipram, a cellular cAMP-elevating agent and antidepressant approved for use in Europe and Japan, effectively inhibits tumor cell proliferation and progression in mice.

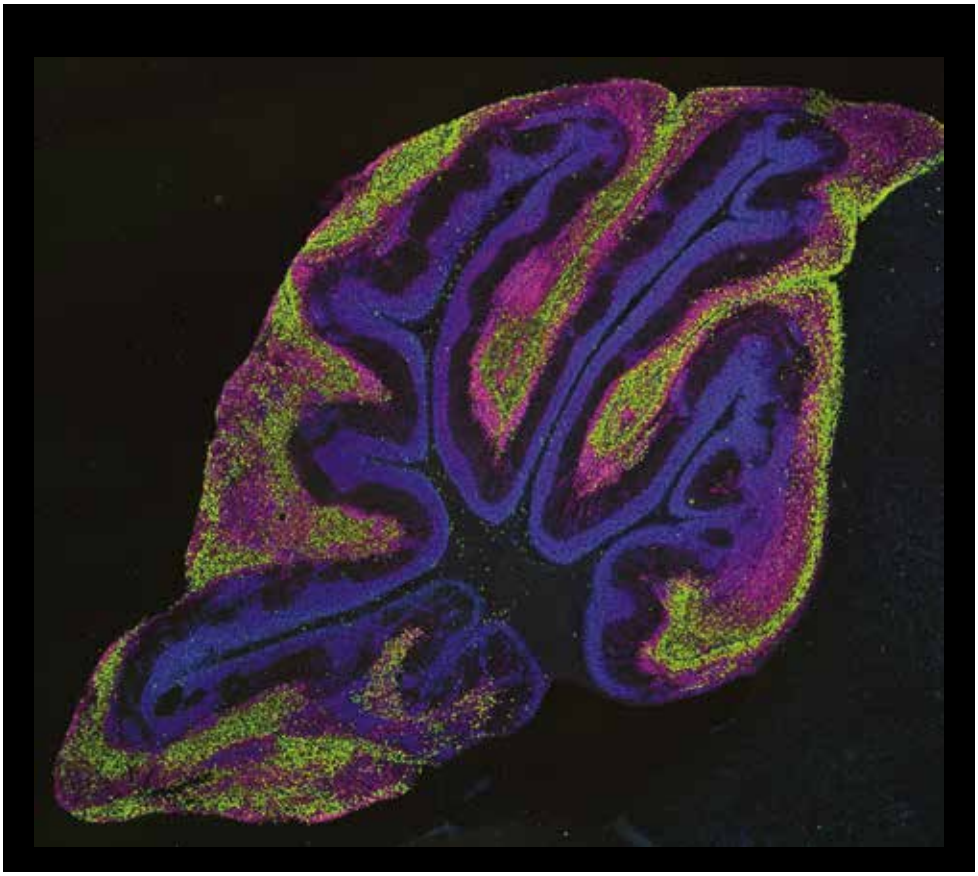
The findings were published in September 2014 in *Nature Medicine*. The study included collaborators from nine medical centers in four countries.

In healthy people, the GNAS gene encodes a Gs-alpha protein, which initiates a molecular signaling cascade that suppresses tumor growth. Mutations disrupting this pathway can lead to rapid cancer cell growth. Lu and colleagues discovered the gene's role while employing a genome-wide screen to analyze childhood brain tumor samples.

In a line of mice bred to lack the GNAS gene, medulloblastomas shrank when given Rolipram. The researchers believe the drug restores the Gs-alpha pathway's tumor suppressing power by elevating levels of the signaling molecule cAMP.

"Many chemotherapies become ineffective as soon as the surface receptors they target change, but this drug may help to get inside the cells by targeting a signaling juncture downstream to overcome the drug resistance," Lu says.

Rolipram is only one drug affecting one part of the Gs-alpha signaling pathway. Lu and colleagues are working to identify other genes and related markers along the pathway. It may be that other drugs acting at other points will prove to be even more effective.



This confocal microscope image of the mouse cerebellum from Gnas mutants is immunostained to show tumor cells (in purple), rapidly dividing tumor cells (in yellow) and granule neurons (in blue). A study published in *Nature Medicine* reveals that treatment with the anti-depressant Rolipram can suppress aggressive sonic hedgehog (SHH)-driven medulloblastomas.

Clot-Stabilizing Enzyme Heals Colitis Damage in Mice and Shows Potential Wider Applications

Joseph Palumbo, MD

CANCER AND BLOOD DISEASES INSTITUTE RESEARCH AND TRAINING DETAILS

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Andersson C, Kvist PH, McElhinney K, Baylis R, Gram LK, Pelzer H, Lauritzen B, Holm TL, Hogan S, Wu D, Turpin B, Miller W, Palumbo JS. Factor XIII Transglutaminase Supports the Resolution of Mucosal Damage in Experimental Colitis. *PLoS One*. 2015;10(6):e0128113.

PUBLISHED JUNE 22, 2015
PLOS ONE

The thrombin-activated transglutaminase factor XIII (FXIII) plays an important supportive role in the repair of colitis-induced mucosal damage in mice, according to research led by Joseph Palumbo, MD, a scientist in the Cancer and Blood Diseases Institute.

FXIII is best known as the enzyme that stabilizes fibrin clots. However, new findings published June 22, 2015, in *PLOS ONE* demonstrate that FXIII also plays a larger-than-expected role in tissue regeneration.

"Until our published report, the only direct evidence for a contribution of FXIII to tissue remodeling was for incisional skin wounds," Palumbo says. "Our findings illustrate the potential to utilize FXIII to resolve a wider range of injuries."

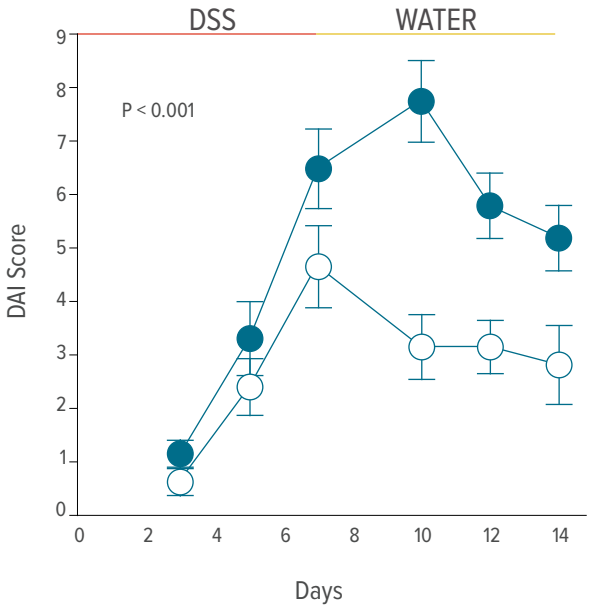
Palumbo, in collaboration with Novo Nordisk scientists Christina Andersson and Brian Lauritzen, evaluated how colitis-challenged mice responded when treated with recombinant human FXIII-A (rFXIII). They found that wildtype (WT) mice and mice genetically bred to lack the FXIII enzyme developed comparable mucosal damage when challenged with dextran sulfate sodium (DSS) to induce colitis symptoms. However, the FXIII-deficient mice failed to resolve the damage after DSS was withdrawn.

Treating mice with rFXIII significantly mitigated the clinical signs of colitis (e.g., weight loss, intestinal bleeding, diarrhea) while also largely resolving mucosal ulceration. Most strikingly, the benefit was not limited to FXIII-deficient animals. Control mice with normal FXIII gene expression also demonstrated a dramatic improvement in mucosal repair when treated with rFXIII following colitis challenge.

Further research is needed to determine the ultimate clinical utility of FXIII in inflammatory bowel disease (IBD). However, the impact of this work may extend beyond IBD.

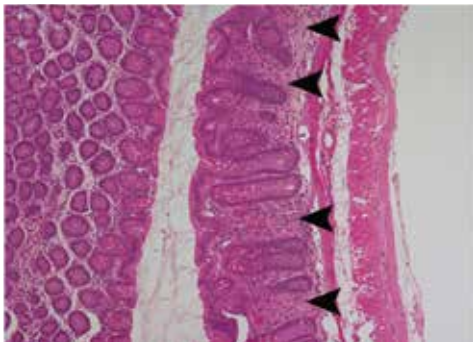
"For example, Matthew Flick in Experimental Hematology has published work detailing the contribution of FXIII to inflammatory arthritis pathogenesis, and Eric Mullins in Hematology has findings suggesting FXIII is linked to neuroinflammatory disease," Palumbo says. "Furthermore, FXIII may play a fundamental role in cardiac tissue repair, another area of intense interest for our group."

A



The transglutaminase factor XIII (FXIII) plays a significant role in mucosal tissue regeneration. Image (A) shows a comparison of Disease Activity Index (DAI), a semiquantitative score of colitis severity based on multiple clinical metrics, in mice challenged with dextran sodium sulfate (DSS) for seven days to induce colitis, then allowed to recover for seven days. Note that mice treated with rFXIII (open circles) showed dramatic improvement in DAI compared to vehicle-treated control mice (closed circles). Image (B) shows colon tissue harvested from a vehicle-treated control mouse at the end of the 14-day experiment. Large remaining areas of inflammatory crypt spacing (arrowheads) demonstrate incomplete mucosal healing. In contrast, image (C) shows that mice treated with rFXIII exhibit near-complete mucosal healing at this time point.

B



C