Institute Summary

RESEARCH AND TRAINING DETAILS

<table>
<thead>
<tr>
<th>Description</th>
<th>Number</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of Faculty</td>
<td>78</td>
</tr>
<tr>
<td>Number of Joint Appointment Faculty</td>
<td>24</td>
</tr>
<tr>
<td>Number of Research Fellows</td>
<td>47</td>
</tr>
<tr>
<td>Number of Research Students</td>
<td>18</td>
</tr>
<tr>
<td>Number of Support Personnel</td>
<td>330</td>
</tr>
<tr>
<td>Direct Annual Grant Support</td>
<td>$17,216,928</td>
</tr>
<tr>
<td>Direct Annual Industry Support</td>
<td>$1,080,971</td>
</tr>
<tr>
<td>Peer Reviewed Publications</td>
<td>161</td>
</tr>
</tbody>
</table>

CLINICAL ACTIVITIES AND TRAINING

<table>
<thead>
<tr>
<th>Description</th>
<th>Number</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of Clinical Staff</td>
<td>13</td>
</tr>
<tr>
<td>Number of Staff Physicians</td>
<td>11</td>
</tr>
<tr>
<td>Number of Clinical Fellows</td>
<td>16</td>
</tr>
<tr>
<td>Number of Other Students</td>
<td>13</td>
</tr>
<tr>
<td>Inpatient Encounters</td>
<td>22,794</td>
</tr>
<tr>
<td>Outpatient Encounters</td>
<td>19,111</td>
</tr>
</tbody>
</table>

Institute Leadership

L to R: R Ware, J Perentesis, Y Zheng

Significant Accomplishments

**Oncology**

**Exome Sequencing Accelerates Personalized Cancer Therapy**

In a landmark investigation, Brian Turpin, DO, presented the Cincinnati Children’s experience in targeted exome sequencing for more than 100 cases of high risk and relapsed childhood cancers and leukemias at the International Society of Pediatric Oncology annual meeting in Hong Kong in 2013. This work represents the first and largest pediatric experience in tumor signatures. The research project has four key implications:

- It identified mutations in more than 70 percent of patient tumors, where previous estimates had asserted that only a minority of patients would have alterations.
- Most of the mutations identified are targetable by new small-molecule anticancer drugs available or in development.
- Tumor mutations did not track with tumor histology, which suggests a new approach for treating patients by genetic signature.
- Drug-resistant pediatric tumors shared targetable genomic alterations common with aggressive adult cancers – a previously unappreciated observation.

This work forms a foundation for personalized precision therapy of high-risk and relapsed pediatric cancers,
augmenting our role as the nation’s largest center for new anticancer drug development in children.

**Experimental Hematology and Cancer Biology**

**A Major Step Forward in Rejuvenating Hematopoietic Stem Cells**

Many tissues in the human body require continuous replenishment from stem cells, but as people age their stem cells begin to decline in function. Now a study led by a Cincinnati Children’s scientist has revealed a critical mechanism involved in this aspect of aging. The regeneration of haematopoietic stem cells (HSCs), which produce all types of blood cells, appears to be controlled by a shift from canonical to non-canonical Wnt signaling due to elevated expression of Wnt5a. The altered signaling triggers a cascade of events that erodes the regenerative capacity of HSCs. Controlling this signaling circuit could help refresh aged or diseased blood stem cells, which in turn could have significant impact on a variety of diseases. The study, published in *Nature*, was led by Hartmut Geiger, PhD, and included a team of scientists from Cincinnati Children’s and the University of Ulm in Germany.

**Novel Therapy for Myelodysplastic Syndrome**

The lab of Daniel Starczynowski, PhD, has identified a potential new approach to treating a collection of bone marrow disorders known as myelodyplastic syndromes (MDSs). These disorders arise from defective hematopoietic stem cells or progenitor cells that produce MDS-initiating clone cells. In a paper published in *Cancer Cell*, Starczynowski and colleagues report that the immune-modulating kinase, IRAK1, is overexpressed and hyperactivated in MDSs. Treating MDS clones in the lab with an IRAK1 inhibitor increased cell death among the MDS cells without affecting normal stem cells. They also found that a combined treatment to inhibit IRAK1 and BCL2 was even more effective at eliminating MDS clones. These findings support further investigation of treatments that target IRAK1.

**Hematology**

**Hematology Division Rated ‘Excellent’ for Hemoglobinopathy Research**

The Division of Hematology has received a five-year, $8.9 million “Excellence in Hemoglobinopathy Research Award” from the National Heart, Lung, and Blood Institute. Led by Punam Malik, MD, the grant will focus on mechanisms that lead to cardiac and kidney damage among patients with sickle cell disease. Co-investigators from Hematology include Theodosia Kalfa, MD, PhD, and Charles Quinn, MD, MS. Other scientists in the Heart Institute also are collaborating.

**Sickle Cell Research Expands in Africa**

The recent recruitment of Russell Ware, MD, PhD, Director of Hematology, and Patrick McGann, MD, MS, is rapidly expanding the role of Cincinnati Children’s in sickle cell research in sub-Saharan Africa. Ware and McGann recently led a highly successful newborn screening initiative in the Republic of Angola. They have since launched several more projects, including a hydroxyurea safety and dosing trial known as Realizing Effectiveness Across Continents with Hydroxyurea (REACH) and the Uganda Sickle Surveillance Study (US3).

**Bone Marrow Transplantation and Immune Deficiency**

**Study Shows Safety in Radiation-Free Transplants**

Our division completed a multi-center study showing that it is safe to perform bone marrow transplants in children with Fanconi Anemia without using radiation. We also established a comprehensive biological sample repository for transplant recipients, storing more than 30,000 samples annually. Our team also has identified
genetic changes that increase the risk of post-transplant thrombotic microangiopathy.

Division Publications


Recombinant soluble CD137 prevents type one diabetes in nonobese diabetic mice. J Autoimmun.


102. Mayes DA, Rizvi TA, Titus-Mitchell H, Oberst R, Ciraolo GM, Vorhees CV, Robinson AP, Miller SD,


116. Pradhan A, Singh TR, Ali AM, Wahengbam K, Meetei AR. Monopolar spindle 1 (MPS1) protein-dependent phosphorylation of RecQ-mediated genome instability protein 2 (RMI2) at serine 112 is


152. Wu J, Patmore DM, Jousmae E, Eaves DW, Breving K, Patel AV, Schwartz EB, Fuchs JR, Cripe TP,


Grants, Contracts, and Industry Agreements

Bone Marrow Transplantation and Immune Deficiency

<table>
<thead>
<tr>
<th>Grant and Contract Awards</th>
<th>Annual Direct</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>DAVIES, S</strong></td>
<td></td>
</tr>
<tr>
<td><strong>Childhood Cancer Survivor Study</strong></td>
<td>National Institutes of Health(St Jude's Children's Hospital)</td>
</tr>
<tr>
<td>U24 CA 055727</td>
<td>12/01/11-11/30/16</td>
</tr>
</tbody>
</table>
COG Group Chair Award - Scientific Leadership
National Institutes of Health (Children's Hospital of Philadelphia)
U10 CA 098543 03/01/12-02/28/17 $11,546

Molecular Epidemiology of Pediatric Germ Cell Tumors
National Institutes of Health (University of Minnesota)
R01 CA 151284 08/10/11-05/31/16 $11,370

Children's Oncology Group Chair - WorkLoad Intensity
National Institutes of Health (Children's Hospital of Philadelphia)
U10 CA 098543 04/13/12-02/28/15 $5,775

FILIPOVICH, A

Gene Therapy for SCID-X1 Using Self-Inactivating (SIN) Gammaretroviral Vector
National Institutes of Health (Children's Hospital Boston)
U01 AI 087628 09/01/10-08/31/15 $54,140

Rare Diseases Clinical Consortia for the Rare Diseases
National Institutes of Health (The Regents of the University of California)
U54 AI 082973 02/09-08/31/14 $1,000

KUMAR, A

The Role of MEIS1 in Hematopoiesis and Hematopoietic Transformation
National Institutes of Health
R01 HL 111192 05/01/13-04/30/17 $271,032

MEHTA, P

Phase I Study of Quercetin for the Treatment of Fanconi Anemia
U.S. Food and Drug Administration
R01 FD 004383 09/16/13-07/31/16 $130,719

MYERS, K

Chemoprevention of Leukemia in a Genetically Susceptible Population
Conquer Cancer Foundation
07/01/12-06/30/15 $62,466

Current Year Direct $726,676

Industry Contracts

GRIMLEY, M

Baxter Healthcare Corporation $1,143
Chimerix Inc $58,769

HARRIS, R

Alexion Pharmaceuticals, Inc $1,155
Experimental Hematology

Grant and Contract Awards

<table>
<thead>
<tr>
<th>Name</th>
<th>Organization and University</th>
<th>Title</th>
<th>Funding Agency</th>
<th>Start Date</th>
<th>End Date</th>
<th>Direct Receipts</th>
</tr>
</thead>
<tbody>
<tr>
<td>AKUNURU, S</td>
<td>National Institutes of Health (University of Cincinnati)</td>
<td>Training Programs in Cancer Therapeutics</td>
<td>National Institutes of Health</td>
<td>10/01/12</td>
<td>08/31/14</td>
<td>$42,862</td>
</tr>
<tr>
<td>CANCELAS-PEREZ, J</td>
<td>The Leukemia and Lymphoma Society</td>
<td>Rational Design of a Vav/Rac Inhibitor as a New Therapy for High-Risk B-ALL</td>
<td>The Leukemia and Lymphoma Society</td>
<td>10/01/12</td>
<td>09/30/15</td>
<td>$180,018</td>
</tr>
<tr>
<td></td>
<td>William Lawrence &amp; Blanche Hughes Foundation</td>
<td>Validation of a Rationally Designed Guanine Nucleotide Exchange Factor Inhibitor in Lymphoblastic Leukemia</td>
<td>William Lawrence &amp; Blanche Hughes Foundation</td>
<td>01/01/14</td>
<td>12/31/16</td>
<td>$70,000</td>
</tr>
<tr>
<td>DEGEN, J</td>
<td>National Institutes of Health (Texas A&amp;M University Health Science Center)</td>
<td>Analysis of Staphylococcus Aureus Host Interactions</td>
<td>National Institutes of Health</td>
<td>09/01/12</td>
<td>08/31/15</td>
<td>$46,736</td>
</tr>
<tr>
<td>DEGEN, J / MALIK P</td>
<td>National Institutes of Health</td>
<td>Hemostatic Factors and Sickle Cell Disease</td>
<td>National Institutes of Health</td>
<td>01/01/12-11/30/16</td>
<td></td>
<td>$245,000</td>
</tr>
<tr>
<td>FILIPPI, M</td>
<td>National Institutes of Health</td>
<td>Regulation of Neutrophil Migration and Polarity</td>
<td>National Institutes of Health</td>
<td>03/01/10</td>
<td>02/28/15</td>
<td>$242,550</td>
</tr>
</tbody>
</table>
Mechanisms Linking the Hemostatic Protease Thrombin to Arthritic Disease
National Institutes of Health
R01 AR 056990 08/10/09-07/31/14 $162,518

Cincinnati Rheumatic Disease Core Center (Core 2)
National Institutes of Health
P30 AR 047363 08/25/11-06/30/16 $45,687

GEIGER, H

Molecular Mechanisms and Therapies for Radiation-Induced Myelodysplastic Syndrome
Edward P Evans Foundation (University of Kentucky)
04/01/12-03/31/17 $181,818

GUO, F

Novel Signaling Function of Cdc42 GTPase in vivo
National Institutes of Health
R01 GM 108661 05/01/14-02/28/18 $175,000

HENNIGAN, R

Regulation of Intracellular Trafficking in NF2
Department of Defense
W81XWH1310136 06/01/13-05/31/16 $133,290

KOMUROV, K

Modeling and Targeting the Hexosamine Pathway in Drug Resistance
Susan G Komen for the Cure
08/01/13-07/31/16 $120,000

LU, Q

A Novel Model of Medulloblastoma to Define Cancer Pathways and Molecular Targets
National Institutes of Health
R01 NS 078092 10/01/13-03/31/17 $349,062

Chromatin Remodeling Control of CNS Myelination and Remyelination
National Institutes of Health
R01 NS 075243 10/01/13-03/31/17 $399,554

Chromatin Remodeling in Oligodendrocyte Myelination and Remyelination
National Multiple Sclerosis Society
RG4568A5T 10/01/13-03/31/14 $31,122

microRNA Control of Myelination and Remyelination in the Central Nervous System
National Multiple Sclerosis Society
RG4727A6T 10/01/13-06/30/15 $148,046

Molecular Mechanisms of Oligodendrocyte Differentiation and Myelination
National Institutes of Health
R01 NS 072427 10/01/13-08/31/15 $138,615

Identification of Novel Small Molecules for CNS Myelin Repair
MALIK, P

Ameliorating Sickle Nephropathy and Pulmonary Hypertension
National Institutes of Health
R34 HL 108752 08/18/11-06/30/14 $142,800

Cincinnati Cell Characterization Core
National Institutes of Health(University of Maryland)
U01 HL 099997 09/01/10-04/30/15 $50,862

Cincinnati Cell Characterization Core (per case reimbursement)
National Institutes of Health(University of Maryland)
U01 HL 099997 05/01/11-04/30/14 $109,291

Cincinnati Center of Excellence in Hemoglobinopathies Research
National Institutes of Health
U01 HL 117709 08/15/13-05/31/18 $1,169,344

Quinn, C Translational Research Skills Development Core $258,435
Kalfa, T Research Project 1 $123,169
Malik, P Research Project 2 $548,319
Quinn, C Research Project 3 $222,743
Kalfa, T Summer Students $40,542

Gene Therapy for Sickle Cell Anemia
Doris Duke Charitable Foundation
09/01/13-08/31/16 $150,000

PLGF-H1F1a-miRNA Axis in Sickle Pulmonary Hypertension
National Institutes of Health(University of Southern California)
R01 HL 111372 01/01/12-12/31/16 $143,001

MULLOY, J

Genotype and Phenotype of Chemoresistant AML
National Institutes of Health
R21 CA 168369 03/01/13-02/28/15 $105,488

Rac Signaling in MLL Leukemia
The Leukemia and Lymphoma Society
07/01/10-06/30/15 $104,762

LSC Mobilization and Differentiation Therapy
Hyundai Hope on Wheels
09/01/13-12/31/15 $250,000

Conferring In Vivo Metabolic Resistance to a Highly Selective Anti-AML Agent
NASSAR, N

**Novel Rationally Designed Ras Inhibitors for B-ALL Multi-Target Therapy**
The Leukemia and Lymphoma Society
10/01/13-09/30/16 $180,018

PAN, D

**Gaucher Disease: Treatment of Neurodegenerative Disease**
National Institutes of Health
R01 NS 086134 09/01/13-05/31/18 $270,887

PANG, Q

**Role of FA Proteins in Hematopoiesis**
National Institutes of Health
R01 HL 076712 04/01/10-03/31/15 $242,550

**Targeted Improvement in Stem Cell Therapy for Leukemia and Bone Marrow Failure Syndromes**
National Institutes of Health
R01 CA 157537 02/01/11-12/31/15 $186,750

PATEL, A

**Identification and Study of Novel Genes Critical to Survival of MPNST Cells**
Ohio State University
06/01/13-05/31/15 $46,092

RATNER, N

**Identification of Molecular and Cellular Contributors to Neurofibroma Formation and Growth**
Department of Defense
W81XWH1210133 07/01/12-06/30/15 $225,000

**Identification of Neurofibroma Growth and Drug Resistance Pathways**
Johns Hopkins University (Neurofibromatosis Therapeutic Acceleration Program)
02/01/14-01/31/16 $65,902

**Mitogenic Activities in Neurofibromatosis**
National Institutes of Health
R01 NS 028840 09/15/11-07/31/16 $223,156

**Neurofibroma Preclinical Therapeutics**
The Children's Tumor Foundation
07/15/13-07/14/16 $287,661

**Ras Proteins in Nerve Tumorigenesis**
National Institutes of Health
R01 NS 083580 04/01/14-03/31/19 $218,750

**Regulation of GCPII for the Diagnosis and Treatment of Neurofibromas**
<table>
<thead>
<tr>
<th>Institutional Name</th>
<th>Start Date</th>
<th>End Date</th>
<th>Funding Agency</th>
<th>Grant Title</th>
<th>Reference Number</th>
<th>Total Funding</th>
</tr>
</thead>
<tbody>
<tr>
<td>John Hopkins University (Neurofibromatosis Therapeutic Acceleration Program)</td>
<td>05/01/14-04/30/15</td>
<td></td>
<td></td>
<td></td>
<td>$33,085</td>
<td></td>
</tr>
<tr>
<td>SAMPSON, L</td>
<td></td>
<td></td>
<td></td>
<td>mTOR Signaling in Murine Intestinal Stem Cell and Progenitor Homeostasis</td>
<td>F32 DK 097879</td>
<td>$55,094</td>
</tr>
<tr>
<td>SPRINGER, M</td>
<td></td>
<td></td>
<td></td>
<td>Training Programs in Cancer Therapeutics</td>
<td>T32 CA 11786</td>
<td>$39,265</td>
</tr>
<tr>
<td>STARCZYNOWSKI, D</td>
<td></td>
<td></td>
<td></td>
<td>Defining the Role and Therapeutic Potential of TNF Receptor-Associated Factor 6 in Myelodysplastic Syndromes</td>
<td>R01 HL 111103</td>
<td>$245,000</td>
</tr>
<tr>
<td>TANDON, P</td>
<td></td>
<td></td>
<td></td>
<td>Characterizing the Role of Specific Ras Proteins in Neurofibroma and MPNST Formation</td>
<td>F32 NS 083249</td>
<td>$49,214</td>
</tr>
<tr>
<td>VARNEY, M</td>
<td></td>
<td></td>
<td></td>
<td>Environmental Carcinogenesis and Mutagensis</td>
<td>T32 ES007250</td>
<td>$46,092</td>
</tr>
<tr>
<td>WU, L</td>
<td></td>
<td></td>
<td></td>
<td>Functional Study of Transcriptional Regulator Sip1 in CNS Myelination and Remyelination</td>
<td>W81XWH1110259</td>
<td>$51,642</td>
</tr>
<tr>
<td>WU, J</td>
<td></td>
<td></td>
<td></td>
<td>STAT3 in Neurofibroma Tumorigenesis and Therapy</td>
<td>W81XWH1110259</td>
<td>$137,415</td>
</tr>
<tr>
<td>Project Title</td>
<td>PI(s)</td>
<td>Institution</td>
<td>Grant Number</td>
<td>Start/End Date</td>
<td>Amount ($)</td>
<td></td>
</tr>
<tr>
<td>------------------------------------------------------------------------------</td>
<td>----------------</td>
<td>--------------------------------------------------</td>
<td>--------------</td>
<td>------------------------</td>
<td>------------</td>
<td></td>
</tr>
<tr>
<td>A Non-Myeloablative Conditioning Regimen for Hematopoietic Stem Cell Transplant</td>
<td>ZHENG, Y</td>
<td>National Institutes of Health</td>
<td>R34 HL117576</td>
<td>02/17/14-02/16/15</td>
<td>$86,732</td>
<td></td>
</tr>
<tr>
<td>Therapeutic Targeting of LARG-RhoA-ROCK Signaling Axis in Childhood Leukemia</td>
<td>ZHENG, Y, GEIGER, H</td>
<td>Alex's Lemonade Stand Foundation</td>
<td></td>
<td>07/01/13-06/30/15</td>
<td>$125,000</td>
<td></td>
</tr>
<tr>
<td>Lineage Determination and Tissue Homeostasis in the Aged Hematopoietic System</td>
<td>ZHENG, Y, GEIGER, H</td>
<td>National Institutes of Health</td>
<td>R01 AG 040118</td>
<td>08/01/11-07/31/16</td>
<td>$212,625</td>
<td></td>
</tr>
<tr>
<td>Targeting Cdc42 in Leukemia Stem Cells</td>
<td>ZHENG, Y, MULLOY J</td>
<td>National Institutes of Health</td>
<td>R01 CA 150547</td>
<td>03/10/10-01/31/15</td>
<td>$195,237</td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>$8,832,677</td>
<td></td>
</tr>
<tr>
<td>Current Year Direct Receipts</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>$190,451</td>
<td></td>
</tr>
</tbody>
</table>

**Industry Contracts**

<table>
<thead>
<tr>
<th>Company</th>
<th>Amount ($)</th>
</tr>
</thead>
<tbody>
<tr>
<td>FLICK, M</td>
<td>$115,747</td>
</tr>
<tr>
<td>GRASSMAN, E</td>
<td>$34,010</td>
</tr>
<tr>
<td>MULLOY, J</td>
<td>$7,261</td>
</tr>
<tr>
<td>STARCZYNOWSKI, D</td>
<td>$33,433</td>
</tr>
</tbody>
</table>

**Total**

$9,023,128

Hematology
<table>
<thead>
<tr>
<th>Name</th>
<th>Project Description</th>
<th>Institution</th>
<th>Start Date</th>
<th>End Date</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>ADAMS, G</td>
<td>Thrombin Promotes Prostate Cancer Progression</td>
<td>The Ohio State University Research Foundation</td>
<td>07/01/13-06/30/15</td>
<td></td>
<td>$41,634</td>
</tr>
<tr>
<td>GRUPPO, R</td>
<td>Hemophilia And Thrombosis Center</td>
<td>Cascade Hemophilia Consortium(Hemophilia Foundation of Michigan)</td>
<td>06/01/13-05/31/15</td>
<td></td>
<td>$114,250</td>
</tr>
<tr>
<td></td>
<td>Hemophilia Comprehensive Care</td>
<td>Maternal and Child Health Bureau(Hemophilia Foundation of Michigan)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Public Health Surveillance for the Prevention of Complications of Bleeding and Clotting Disorders</td>
<td>Centers for Disease Control &amp; Prevention(Hemophilia Foundation of Michigan)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>My Life, Our Future: A Hemophilia Genotyping Initiative</td>
<td>American Thrombosis &amp; Hemostasis Network</td>
<td>02/01/14-01/31/15</td>
<td></td>
<td>$3,500</td>
</tr>
<tr>
<td></td>
<td>Hemophilia Inhibitor Pup Study (per capita)</td>
<td>The University of Texas Health Science Center at Houston</td>
<td></td>
<td></td>
<td>$8,540</td>
</tr>
<tr>
<td>KALFA, T</td>
<td>Rho GTPases in Terminal Erythroid Maturation</td>
<td>National Institutes of Health</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Cincinnati Center of Excellence in Hemoglobinopathies Research - Research Project 1:</td>
<td>National Institutes of Health</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Signaling Pathways that Regulate ROS Production in Sickle RBCs and Contribution to</td>
<td>National Institutes of Health</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Hemolysis, SN and Cardiac Pathology</td>
<td>National Institutes of Health</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Cincinnati Center of Excellence in Hemoglobinopathies Research - Summer Students</td>
<td>National Institutes of Health</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MULLINS, E</td>
<td>Mechanisms Linking Hemostatic Factors to Neuroinflammatory Disease</td>
<td>National Institutes of Health</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>QUINN, C</td>
<td>A Controlled Clinical Trial of Regadenoson in Sickle Cell Anemia</td>
<td>National Institutes of Health</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
P50 HL 110790 07/01/13-06/30/17 $78,470

**PFAST: Patent Foramen Ovale and Stroke in Sickle Cell Disease (per capita)**

Doris Duke Charitable Foundation (The University of Texas Southwestern Medical Center)

DDCF #2009091 02/15/11 -04/30/14 $10,449

**Cincinnati Center of Excellence in Hemoglobinopathies Research - Translational Research Skills Development Core**

National Institutes of Health

U01 HL 117709 08/15/13-05/31/18 $258,435

**Cincinnati Center of Excellence in Hemoglobinopathies Research - Research Project 3 - Novel Cardiac Magnetic Resonance Imaging to Define a Unique Restrictive Cardiomyopathy in Sickle Cell Disease**

National Institutes of Health

U01 HL 117709 08/15/13-05/31/18 $222,743

**SHOOK, L**

**Cincinnati Sickle Cell Newborn Screening Network**

Health Resources & Services Administration

U38 MC 22218 06/01/11-05/31/15 $319,804

**Cincinnati Sickle Cell Project**

Ohio Department of Health

07/01/98-06/30/14 $123,469

**WARE, R**

**Accurate and Inexpensive Point-of-Care Diagnosis of Sickle Cell Anemia**

Doris Duke Charitable Foundation (Rice University)

09/01/13-08/31/16 $45,305

**Baby Hug Follow-up Study II Core Laboratory**

National Institutes of Health (Clinical Trials & Surveys Corp)

HHSN268201200023C 07/01/13-12/31/16 $2,101

**Genetic Predictors of Cerebrovascular Disease in Sickle Cell Anemia**

Doris Duke Charitable Foundation

08/01/13-07/31/14 $178,722

**RH Genotyping of Patient with Sickle Cell Anemia from a Multi-Center Study (SWiTCH Trial) and Correlation with Alloimmunization Following Blood Transfusion**

Doris Duke Charitable Foundation (New York University School of Medicine)

01/01/14-12/31/14 $9,259

**Sparing Conversion to Abnormal TCD Elevation**

National Institutes of Health

R01 HL 098239 12/01/13-11/30/14 $533,037

**TCD with Transfusions Changing to Hydroxyurea**

National Institutes of Health

R01 HL 095647 08/01/13-07/31/14 $3,033,934

**Endothelialized Microfluidics for Sickle Cell Disease Research & Drug Discovery**

National Institutes of Health (Emory University)

R01 HL 121264 01/01/14-12/31/18 $11,593
### Industry Contracts

<table>
<thead>
<tr>
<th>Name</th>
<th>Company</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>GRUPPO, R</td>
<td>Alexion Pharmaceuticals, Inc</td>
<td>$10,128</td>
</tr>
<tr>
<td></td>
<td>Baxter Healthcare Corporate</td>
<td>$98,792</td>
</tr>
<tr>
<td></td>
<td>Bayer HealthCare Pharmaceuticals, Inc</td>
<td>$36,723</td>
</tr>
<tr>
<td></td>
<td>Biogen Idec MA Inc.</td>
<td>$42,450</td>
</tr>
<tr>
<td></td>
<td>Boehringer Ingelheim Pharmaceuticals</td>
<td>$14,650</td>
</tr>
<tr>
<td></td>
<td>Grifols, Inc</td>
<td>$1,000</td>
</tr>
<tr>
<td></td>
<td>Novo Nordisk Pharmaceuticals</td>
<td>$38,836</td>
</tr>
<tr>
<td></td>
<td>Pfizer, Inc</td>
<td>$97,576</td>
</tr>
<tr>
<td></td>
<td>Rho, Inc.</td>
<td>$500</td>
</tr>
<tr>
<td>KALFA, T</td>
<td>Baxter</td>
<td>$750</td>
</tr>
<tr>
<td>KALINYAK, K</td>
<td>Novartis Pharmaceuticals</td>
<td>$4,505</td>
</tr>
<tr>
<td>PALUMBO, J</td>
<td>Novo Nordisk Pharmaceuticals</td>
<td>$43,187</td>
</tr>
<tr>
<td>QUINN, C</td>
<td>Amgen, Inc.</td>
<td>$51,770</td>
</tr>
<tr>
<td></td>
<td>Eli Lilly and Company</td>
<td>$9,500</td>
</tr>
<tr>
<td></td>
<td>GlycoMimetics, Inc</td>
<td>$5,197</td>
</tr>
<tr>
<td></td>
<td>Mast Therapeutics, Inc</td>
<td>$15,015</td>
</tr>
<tr>
<td>WARE, R</td>
<td>Bristol-Myers Squibb</td>
<td>$20,000</td>
</tr>
</tbody>
</table>

### Current Year Direct Receipts

<table>
<thead>
<tr>
<th>Name</th>
<th>Company</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Current Year Direct</td>
<td>$490,579</td>
</tr>
<tr>
<td></td>
<td>Total</td>
<td>$6,049,810</td>
</tr>
</tbody>
</table>

### Oncology

<table>
<thead>
<tr>
<th>Name</th>
<th>Grant and Contract Awards</th>
<th>Annual Direct</th>
</tr>
</thead>
<tbody>
<tr>
<td>CHLON, T</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Program/Consortium</td>
<td>Investigator</td>
<td>Title</td>
</tr>
<tr>
<td>-------------------</td>
<td>-------------</td>
<td>-------</td>
</tr>
<tr>
<td>Pelotonia Fellowship Program</td>
<td></td>
<td></td>
</tr>
<tr>
<td>CHOW, L</td>
<td>Molecular Targeting of High-Grade Astrocytoma</td>
<td>The Sontag Foundation</td>
</tr>
<tr>
<td>CHOW, L</td>
<td>Molecular Targeting of Pediatric High-Grade Glioma</td>
<td>St. Baldrick's Foundation</td>
</tr>
<tr>
<td>DASGUPTA, B</td>
<td>Regulation of Forebrain Neurogenesis by the Energy Sensor AMP Kinase</td>
<td>National Institutes of Health</td>
</tr>
<tr>
<td>FOULEDI, M</td>
<td>NIH COG Study Chair</td>
<td>National Institutes of Health (Children's Hospital of Philadelphia)</td>
</tr>
<tr>
<td>FOULEDI, M</td>
<td>Establishment of an International Diffuse Intrinsic Pontine Glioma (DIPG) Registry</td>
<td>The Cure Starts Now Foundation</td>
</tr>
<tr>
<td>FOULEDI, M</td>
<td>The Pediatric Brain Tumor Consortium - Per Patient</td>
<td>National Institutes of Health (St. Jude Children's Hospital)</td>
</tr>
<tr>
<td>FOULEDI, M</td>
<td>The Pediatric Brain Tumor Consortium</td>
<td>National Institutes of Health (St. Jude Children's Hospital)</td>
</tr>
<tr>
<td>GELLER, J</td>
<td>NIH COG Phase 1 Study Chair</td>
<td>National Institutes of Health (Children's Hospital of Philadelphia)</td>
</tr>
<tr>
<td>GELLER, J</td>
<td>Epigenetic and Clinical Impact of SMARCB1 Loss in Cancer</td>
<td>National Institutes of Health (Children's Memorial Hospital)</td>
</tr>
<tr>
<td>LEE, J</td>
<td>Proliferation Control of DIPG Cells by AMP Linase Inhibition</td>
<td>Joshua's Wish</td>
</tr>
<tr>
<td>Grant Title</td>
<td>Institution</td>
<td>Start Date</td>
</tr>
<tr>
<td>----------------------------------------------------------------------------</td>
<td>--------------------------------------------------</td>
<td>------------</td>
</tr>
<tr>
<td>Targeted Inhibition of Cdc42 GTPase in the Acute Myeloid Leukemia Stem Cell</td>
<td>St. Baldrick's Foundation</td>
<td>07/01/13</td>
</tr>
<tr>
<td>Children's Oncology Group Chair - Workload Intensity</td>
<td>National Institutes of Health</td>
<td>03/01/12</td>
</tr>
<tr>
<td>Children's Oncology Group Chair - per patient</td>
<td>National Institutes of Health</td>
<td>03/01/12</td>
</tr>
<tr>
<td>CHOP/COG ADVL0921</td>
<td>Millennium Pharmaceuticals</td>
<td>01/01/14</td>
</tr>
<tr>
<td>COG Supplemental Reimbursement</td>
<td>St. Baldrick's Foundation</td>
<td>03/01/12</td>
</tr>
<tr>
<td>CHOP/COG AALL0932</td>
<td>National Institutes of Health</td>
<td>03/01/12</td>
</tr>
<tr>
<td>Pediatric Phase I - Pilot Consortium (per case)</td>
<td>National Institutes of Health</td>
<td>07/24/12</td>
</tr>
<tr>
<td>Pediatric Phase I Scientific Leadership</td>
<td>National Institutes of Health</td>
<td>09/26/12</td>
</tr>
<tr>
<td>COG ADVL1213</td>
<td>Morphotek</td>
<td>01/01/14</td>
</tr>
<tr>
<td>COG Community Clinical Oncology Program</td>
<td>National Institutes of Health</td>
<td>08/01/13</td>
</tr>
<tr>
<td>COG Cancer Trial Support Unit</td>
<td>National Institutes of Health</td>
<td>03/01/14</td>
</tr>
<tr>
<td>COG AALL1131</td>
<td>National Institutes of Health</td>
<td>01/01/14</td>
</tr>
<tr>
<td>COG AALL1121</td>
<td>Amgen</td>
<td>05/01/12</td>
</tr>
</tbody>
</table>
Genetic Model of Cytarabine Sensitivity in Children with AML
American Association for Cancer Research
07/01/12-06/30/14 $50,000

PRIVETTE, L

Cincinnati Interdisciplinary Women’s Health Research Career Training Grant
National Institutes of Health(University of Cincinnati)
K12 HD 051953 07/01/12-06/30/14 $82,385

Defining the Role of the DEK Oncogene in Breast Cancer Stem Cell Tumorigenicity And Pre-Clinical Testing of Therapeutic DEK Targeting Strategies
Ride Cincinnati Foundation
06/01/14-05/31/15 $40,000

RAYES, A

Mobilization with CASIN as a Preparative Regimen in Bone Marrow Transplant Therapy
Cancer Free Kids
06/01/14-05/31/15 $30,000

ROMICK-ROSENDALE, L

Environmental Carcinogenesis and Mutagenesis
National Institutes of Health(University of Cincinnati)
T32 ES 007250 09/01/12-06/30/14 $41,364

WEISS, B

Children’s Oncology Group Chair
National Institutes of Health(Children’s Hospital of Philadelphia)
U10 CA 098543 04/13/13-02/28/15 $11,550

Phase I Study of MEK Inhibitor AZD6244 in NF1 with Plexiforms
Children’s Tumor Foundation(Children’s Hospital of Philadelphia)
02/01/12-04/30/15 $22,952

WELLS, S

Fanconi Anemia and HPV Transformation
National Institutes of Health
R01 CA 102357 09/28/09-08/31/14 $180,324

Role and Regulation of the Human DEK Proto-Oncogene
National Institutes of Health
R01 CA 116316 09/05/12-06/30/17 $163,859

Targeting the Ron-DEK Signaling Axis in Breast Cancer
Department of Defense
W81XWH-12-1-0194 09/01/12-08/31/14 $125,000

WILLIAMS, J
## ETV2 Role in Tumor-Induced Lymphangiogenesis, A Putative Therapeutic Target

**St. Baldrick's Foundation**

**07/01/12-06/30/14**

$71,748

### Current Year Direct

<table>
<thead>
<tr>
<th>Industry Contracts</th>
<th>Current Year Direct Receipts</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td><strong>$2,098,344</strong></td>
</tr>
</tbody>
</table>

### Industry Contracts

<table>
<thead>
<tr>
<th>Contractor</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pfizer, Inc.</td>
<td>$120,000</td>
</tr>
<tr>
<td>Pierre Fabre Pharmaceuticals</td>
<td>$5,100</td>
</tr>
<tr>
<td>Genentech, Inc</td>
<td>$11,550</td>
</tr>
<tr>
<td>Novartis Pharmaceuticals</td>
<td>$18,750</td>
</tr>
<tr>
<td>Bayer HealthCare Pharmaceuticals, Inc.</td>
<td>$31,138</td>
</tr>
<tr>
<td>Lilly USA, LLC</td>
<td>$13,650</td>
</tr>
<tr>
<td>Glaxo Smith Kline</td>
<td>$13,500</td>
</tr>
<tr>
<td>Seattle Genetics, Inc.</td>
<td>$39,397</td>
</tr>
<tr>
<td>Sarcoma Alliance for Research through Collaboration</td>
<td>$8,500</td>
</tr>
<tr>
<td>Sarcoma Alliance for Research through Collaboration</td>
<td>$5,236</td>
</tr>
<tr>
<td>Bacterial Robotics, LLC</td>
<td>$4,004</td>
</tr>
<tr>
<td>Novartis Pharmaceuticals</td>
<td>$17,000</td>
</tr>
<tr>
<td>NANT</td>
<td>$5,822</td>
</tr>
</tbody>
</table>

### Current Year Direct Receipts

**$293,647**

**Total**

$2,391,991