The CHOP Cancer Center

Stephen P. Hunger, MD
Distinguished Chair in Pediatrics
Professor of Pediatrics
Chief, Division of Oncology
Director, Center for Childhood Cancer Research
Welcome

Karla Flook
People Against Childhood Cancer

CAC2 Inaugural Board Members, installed October 2013

Vickie Buenger, President
Independent Advocate

Lisa Towry, Secretary
Alex’s Lemonade Stand Foundation

Steve Pessagno, Treasurer
Independent Advocate

Steve Crowley, Communications
Committee Co-Chair
Independent Advocate

Joy Cruse, Meeting Committee
Co-Chair
Team Connor

Angie Giallourakis,
Website Chair Co-Chair
Steven G. AYA Cancer Research
Fund

Trish Kriger, Communications
Committee Co-Chair
The Jeff Gordon Children’s
Foundation

Beth Ann Krimsky, Membership
Committee Co-Chair
I Care, I Cure

Donna Ludwinski, Membership
Committee Co-Chair
Solving Kids Cancer

Suzanne Nixon, Audit
Committee Chair and Meeting
Committee Co-Chair
Noah’s Light Foundation

Tom Pilka, Development
Committee Chair
Independent Advocate

Julie Sutherland,
Website/Communication
Liaison
Make Some Noise: Cure Kids
Cancer Foundation

Tony Stoddard
Gold in September
**CAC2 Member Foundations**

<table>
<thead>
<tr>
<th>Foundation Name</th>
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<td>3/32 Foundation</td>
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<td>Ethan Jostad F</td>
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<td>This Star Won’t Go Out</td>
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<td>TN Cancer Coalition</td>
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<td>Zoe4Life</td>
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Special Thanks

- Alex’s Lemonade Stand Foundation
- Andrew McDonough B+ Foundation
- Arms Wide Open Childhood Cancer Foundation
- Bear Necessities Pediatric Cancer Foundation
- CURE Childhood Cancer
- Curing Kid’s Cancer Foundation
- Dragon Master Foundation
- Gold in September
- I Care I Cure Childhood Cancer Foundation
- Make Some Noise, Cure Kid’s Cancer Foundation
- Open Hands Overflowing Hearts
- Solving Kids Cancer
- TeamConnor Cancer Foundation
- The Evan Foundation
- The Naya Foundation
Children’s Hospital of Philadelphia (CHOP)

- Established in 1855 as the first freestanding Children’s Hospital in the US
- 521 Beds and new outpatient Buerger Center for Advanced Pediatric Medicine
  - 30,000 inpatient admissions
  - 90,000 Emergency Department visits
  - 1.2 million outpatient visits

- Extensive network in Delaware Valley
  - 40 locations
  - 9 Specialty Care Center
  - 3 Ambulatory Surgery Centers

- Key Leadership
  - President and CEO: Madeline Bell
  - Executive VP and Chief Scientific Officer: Bryan Wolf, MD PhD
  - Chairman, Department of Pediatrics: Joseph St. Geme, MD
CHOP Cancer Center

• Includes the entire clinical and research enterprise focused on Pediatric Cancer at CHOP
  – Clinical Program including faculty in associated Divisions and Departments
  – Division of Oncology
  – Center for Childhood Cancer Research (CCCR)

• Stephen Hunger, MD joined CHOP in November 2014 as Chief of the Division of Oncology and Director of the CCCR
Abramson Cancer Center

• Led by Chi Van Dang, MD PhD
  – 10 Associate Directors including Garrett Brodeur (AD for Pediatric Research)

• Funding
  – $167 million direct, including $48 million direct in NCI funding

• Organized into 11 Programs
  – Basic Research (3), Population Sciences (2) and Translational Research (6)
  – Pediatric Cancer Program is one of the Translational Research Programs
    • Co-Directed by Frank Balis and Garrett Brodeur
    • 34 members, $16.5 million annual direct cancer related funding, $2 million NCI funding
    • Some Oncology and CCCR faculty are primary members of other Cancer Center Programs
Division of Oncology

• Leadership
  – Chief: Stephen Hunger
  – Medical Director: Anne Reilly
  – Executive Committee: Hunger, Reilly, Garrett Brodeur and Stephan Grupp

• Faculty
  – 56 members including 49 MDs and 6 PhD clinicians or scientists
    • Instructors: 8 MDs
    • Assistant Professors: 17 MDs and 5 PhDs
    • Associate Professors: 9 MDs
    • Professors: 16 MDs and 1 PhD

• Administration
  – John Simpkins is Administrative Director and Director of Cancer Service Line

• Separate Division of Hematology (Chief: Mortimer Poncz) with 23 faculty members
Center for Childhood Cancer Research (CCCR)

- Established in 2007 and spans the spectrum of basic, translational and clinical research in pediatric cancer
- Leadership
  - Stephen Hunger, MD: Director
  - Frank Balis, MD: Director of Clinical Research
  - Director of Basic Science Research: Open
  - Stephan Grupp, MD PhD: Director of Translational Research
- Membership
  - 58 members from the Division of Oncology
  - 17 members from other Divisions in the Department of Pediatrics (Cardiology, Endocrinology, Genetics, Hematology, and Infectious Diseases) and other Departments (Biostatistics and Epidemiology, Child and Adolescent Psychiatry and Behavioral Sciences, Neurosurgery, Pathology, Radiation Oncology, and Surgery)
- Space
  - 56,000 square feet of wet bench space and adjacent offices occupying the entire 3rd and 4th floors of the Colket Translational Research Building
  - About 22,000 square feet of offices on the 10th floor of CTRB for faculty and clinical research teams
Oncology Clinical Program: Volumes

• One of largest Programs in North America
  – 600 newly diagnosed patients and 500 new consultations/referrals each year

• Inpatient Program (all at CHOP)
  – 50 bed unit (single patient rooms, HEPA filtered)
  – Average Daily Census 46.3 patients
  – # of Oncology patients in the Hospital goes as high as 75-80 counting PICU

• Three sites of outpatient practice; total ~20,000 visits/year
  – Main campus
    • Clinic moved to Buerger Center for Advanced Pediatric Medicine in Oct 2015
  – Specialty Care Centers: 5 day/week outpatient clinics and infusion centers
    • Voorhees Specialty Care Center, Voorhees NJ
      – 20 miles from CHOP
      – 3 MDs and 1 PNP
    • King of Prussia Specialty Care Center, King of Prussia PA
      – 20 miles from CHOP
      – 2 MDs and 1 PNP
Oncology Clinical Program: Sections and Programs

• Administrative oversight (report to Anne Reilly)
  – Inpatient Medical Director (Jason Freedman)
  – Outpatient Medical Director (Susan Rheingold)

• Four separate clinical teams (Sections) with independent inpatient and outpatient clinical services.
  – Blood and Marrow Transplant (Nancy Bunin)
  – Hematologic Malignancies (Richard Aplenc)
  – Neuro-Oncology (Peter Phillips)
  – Solid Tumors (Rochelle Bagatell)

• Three trans-disciplinary programs
  – Behavioral Oncology (Lamia Barakat)
  – Cancer Survivorship (Jill Ginsberg)
  – Developmental Therapeutics (Elizabeth Fox)

• Cancer Cellular Immunotherapy Program (Stephan Grupp) named first CHOP Frontier Program in 2015
Blood & Marrow Transplant Section

- Established 1976 (Allergy/Immunology)
- World leader in next generation sequencing based HLA typing
- Research and clinical focus on graft engineering to reduce the risk of GVHD and maintain GVL – αβT/CD19 depletion

Transplant Activity

<table>
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<th>Year</th>
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<th>Autologous</th>
<th>Allogeneic</th>
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<tr>
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<td>56</td>
<td>19</td>
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<td>104</td>
<td>43</td>
<td>61</td>
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<tr>
<td>PRJ 2015</td>
<td>102</td>
<td>46</td>
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Hematologic Malignancy Section: Research Programs

- COG based clinical and translational research
  - ALL: Hunger, Teachey (AALL1231; T-ALL), Tasian (AALL1531; Ruxotinitib + chemotherapy in newly diagnosed ALL with JAK pathway mutations),
  - AML: Aplenc (AAML1031; phase 3 RCT testing addition of bortezomib)
- CAR T cell therapy
  - CART19 and CART22: Maude with Rheingold providing pre-CART infusion support
  - AML CARs: Tasian, Aplenc
- ALL genomics
  - Hunger leads ALL TARGET Project and SBF Consortium Grant testing targeted therapy for Ph-like ALL
- Leading center for patient derived xenograft models of human leukemia
  - Teachey, Tasian, Maude
- Signal transduction inhibition
  - Teachey, Maude, Tasian
- Clinical and genetic epidemiology
  - Aplenc, Bailey, Kersun, Rheingold, Reilly, Seif
Neuro-Oncology Section

• 2015 volumes
  – 110 New Patient Brain Tumor Resections
  – 153 New Brain Tumor Consultation Referrals
  – >500 Neurofibromatosis Patients
  – 78 Brain/Spinal Cord Proton Therapy Patients

• Major unique programs
  – World’s largest pediatric proton therapy program
  – Lead institution for Children’s Brain Tumor Tissue Consortium
    • 7 member institutions
    • Over 6000 biospecimens
    • Over 1500 subjects with clinical data
    • Next generation sequencing of defined patient cohorts
Solid Tumor Section

- Provide care and conduct research in all types of pediatric solid tumors
- Leadership in development of novel therapies for neuroblastoma
  - $^{131}$I-MIBG therapy
  - Tandem autologous transplant
  - ALK inhibitor therapy (crizotinib and 2nd generation inhibitors)
  - Precision medicine therapies
- Broad and deep range of research
  - **Bagatell**: Clinical trials, database studies
  - **Balis**: Dinutuximab PK
  - **Brodeur**: Roles of CHD5 and TRK, nanoparticle drug delivery
  - **Fox**: Early phase trials
  - **Grupp**: GD2 CAR T cell therapy
  - **Hogarty**: ARID alterations, roles of Bcl2 family proteins and polyamines
  - **Maris**: Genetic landscape at dx/relapse, clonal evolution; immunogenomics (SU2C)
  - **Mosse**: Genetic predisposition, targeting ALK, drug development
  - **Mostoufi-Moab**: Bone health in survivors
Programs: Behavioral Oncology

- Led by Lamia Barakat, PhD
- 3 additional Assistant Professor PhD Psychologists
- Research intensive group with 1.7 clinical FTEs among the 4 faculty members
- Apply behavioral translational research to improve outcomes in pediatric cancer across the continuum of care with an emphasis on neurodevelopmental and psychosocial processes of risk and resilience
  - Develop, evaluate and disseminate contextual models to inform psycho-social research and clinical care
  - Develop, evaluate and disseminate psychosocial screening measures, assessments and interventions to prevent and ameliorate cognitive and psychosocial challenges
  - Provide leadership in behavioral translational research, clinical and research training and staff support
  - Collaborate with CHOP Cancer Center and CCCR members, the ACC, the COG, and others nationally and internationally
Programs: Cancer Survivorship

- Established by Anna Meadows
- Led by Jill Ginsberg since 2008
- Volumes
  - 600+ survivors seen annually in Survivorship clinic
- Major activities and areas for research
  - General Survivorship Clinic
  - The Multidisciplinary Survivorship Clinic
    - Dedicated to most complicated survivors
    - Includes MDs from 7 specialties/disciplines
  - Young Adult Transition Program
  - Fertility Preservation
    - Major area of clinical and research focus
Programs: Developmental Therapeutics (DT)

- Led by Elizabeth Fox
- Local activities
  - One of 4 ALSF Centers of Excellence (COE)
  - Leads development of investigator-initiated and pharma trials
    - Phase I trial of trametinib and dabrafenib uses PK endpoints and is one of 1st pediatric trials to combine 2 novel agents
    - Phase I trial of entrectinib translating findings from the Brodeur lab to a clinical trial (ALSF COE study)
- Robust cancer pharmacology lab led by Frank Balis
  - PK/PD studies of dinutuximab informing design of new trials
- National activities: COG
  - Program members play leadership roles in COG Phase I Consortium
    - Vice Chairs: Elizabeth Fox and Yael Mosse
    - Chairs of active DVL trials: Kristina Cole, Yael Mosse, Susan Rheingold
  - CHOP is #1 accruing center to COG DVL studies
    - 40% of these enrollments are outside referrals
Focused Clinical Programs

- Adolescent and Young Adult Oncology
- Cancer Predisposition
- Fertility Preservation
- Hemophagocytic Lymphohistiocytosis
- Langerhans Cell Histiocytosis
- High Risk and Relapsed Neuroblastoma
- Neurofibromatosis
- Pigmented Lesions
- Retinoblastoma
- Thyroid Cancer
Linked Programs Within CHOP

- Cancer Genomic Diagnostics
- Childhood Brain Tumor Tissue Consortium
- Diagnostic Pathology
- Division of Cancer Pathobiology
- Neurosurgery
- Orthopedic Oncology
- Pediatric Advanced Care Team (Palliative Care Medicine)
- Pediatric Surgery
- Radiation Oncology
CHOP Cancer Center: Clinical Trials Portfolio

• 163 open clinical trials
  – 87 interventional, 42 observational, 34 ancillary/correlative

• Significant recent growth in open protocols
  – 36% growth from 2012 to 2015 (16.7% annualized growth rate)

• 1845 enrollments in 2014
  – 245 interventional (89 COG and 156 non-COG)
  – 1126 observational
  – 476 ancillary/correlative

• Oncology accounts for one-third of all clinical trials open at CHOP
Pediatric Cancer: Major Trends

• Precision cancer medicine
• Cancer Predisposition
• Cancer Immunotherapy
The goal in precision cancer medicine is to improve cure rates and decrease toxicities by identifying the specific genes, proteins and pathways responsible for malignant transformation or progression of individual cancers, and utilize therapies that target these underlying features.

Major challenges in moving from research and focused implementation in small high-risk patient subsets to universal use of host/tumor genomic profiling to refine the care of every child.

Technologies will evolve rapidly from gene panels to unbiased whole exome, whole genome, and whole transcriptome (RNAseq) profiling.
Cancer Predisposition

- Today counseling and testing are focused largely on patient subsets that meet specific criteria of syndromes or are at high risk based on personal and/or family history to have germline alterations. Collectively these account for 10-15% of pediatric cancer patients
  - Retinoblastoma, multiple primary cancers, adrenocortical carcinoma, etc
- Physicians struggle to identify all patients that meet these criteria consistently and refer them for consultation/testing
- Emerging data suggest that 10-15% of children with cancer who don’t have obvious predisposing factors have germline mutations in “cancer genes”
- In the near future, genetic counseling and potential germline testing should be offered to every pediatric cancer patient
  - Major challenge to current infrastructures
Cancer Immunotherapy

• Monoclonal antibody based therapies
  – “Naked” antibodies
  – Antibodies conjugated to drugs or radioisotopes
• Cellular immunotherapies
• Drugs targeting pathways involved in immune recognition and destruction of cancer cells
  – Checkpoint inhibitors are rapidly revolutionizing treatment of many adult cancers, but are still very early in pediatric development
CHOP Cancer Center: Cutting Edge Programs

• Thyroid Cancer
• NEPENTHE trial for relapsed neuroblastoma
• Immunotherapy with chimeric antigen receptor (CAR) modified T-cells
Introduction and Program Members

Program Members

• **Andrew J Bauer, MD**
  - *Director* (Endocrinology)
  - Kenya Linton, RN
  - Sandy Tomlinson, RN
  - Jennifer Hufford, RN – research

• **Goli Mostoufi-Moab, MD MSCE**
  - *Co-Director* (Oncology & Endocrinology)

• **Scott Adzick, MD, MMM**
  - Surgeon-in-Chief, CHOP

• **Ken Kazahaya, MD, MBA**
  - Associate Director, Division of OTO
The Thyroid Center

- Evaluate ~ 200 new thyroid nodules/year
- Perform ~ 50 FNA/year
- Diagnose ~ 30 new thyroid cancer/year
- Perform ~ 80 thyroid surgeries/year
- Actively manage about 150 thyroid cancer patients
- > 3500 patients evaluated in the Thyroid Center since inaugural clinic 11/2009
The Thyroid Center

Thyroid Cancer 2009-2013
The Thyroid Center
A better way forward

- **MGH/Boston** – 171 thyroid surgeries/22 years; 85 TTx, 39 LND
  Scholz et al. J Ped Surg 2011

- **MD Anderson** - 74 pediatric patients for TTx in 10 years, 39 with DTC
  Morris et al. Surg 2012

- **Mayo Clinic** - (including up to age 20yrs) - 215 patients over 68 years
  Hay et al. World J Surg 2010

- **Toronto Hospital for Sick Children** - 61 cases over 23 years
  Gorman et al. Thyroid 2010

- **CHOP** - ~5 years (2010 -> Aug 2015*)
  - ~350 Thyroid surgeries
  - 150 Thyroid carcinoma
CHOP Cancer Center: Cutting Edge Programs

- Thyroid Cancer
- NEPENTHE trial for relapsed neuroblastoma
- Immunotherapy with chimeric antigen receptor (CAR) modified T-cells
Development of a Next Generation Sequencing (NGS)-based Biomarker Defined Clinical Trial for Relapsed Neuroblastoma

- Sufficient number of biomarker-drug matches nominated and validated both *in vitro* and *in vivo* to distinguish patients likely to benefit from specific drugs

- Sufficient equipoise in the potential benefit of biopsy at time of relapse

- Sufficient interest in industry to support a multi-IND pediatric clinical trial
Next generation PERSONalized Neuroblastoma THERapy (NEPENTHE)

Relapsed or primary refractory high-risk neuroblastoma

Screen for Part 1

Biopsy of target lesion

Quality control and submit for sequencing

Next Generation Sequencing Results

Screen for Part 2

Biomarker-defined therapeutic Group assignment

- Group 1
  Ceritinib + Ribociclib
  Phase 1/Expansion

- Group 2A
  Trametinib Expansion

- Group 3
  HDM201
  Phase 1/Expansion

- No biomarker match
  Not eligible for Part 2

- Group 2B
  Trametinib + Ribociclib
  Phase 1/Expansion

Leaders: Yael Mosse and John Maris

• Study will use genomic information generated in real time for selection of investigational therapy.
• Primary objectives: safety and ORR within context of a phase 1/1b biomarker-driven trial
• Opportunity to define genomic landscape or relapsed NB, and to determine frequency by which a drug-target match leads to objective benefit
• Correlative biology studies will include
  • Serial detection of mutations in circulating cfDNA shed from tumor cells
  • Generate PDX models
  • Define clonal evolution
CHOP Cancer Center: Cutting Edge Programs

- Thyroid Cancer
- NEPENTHE trial for relapsed neuroblastoma
- Immunotherapy with chimeric antigen receptor (CAR) modified T-cells
CHIMERIC ANTIGEN RECEPTOR (CAR)

CD19+ tumor

MHC-independent antigen engagement and induction of signalling

APC

CD28

4-1BB (CD137)

CD3 zeta

Proliferation, cytokine production, CTL function, tumor lysis
Redirecting T cell Specificity in CTL019 cells

Goals for modern, highly active cell therapy:

• **Proliferation** – high level of in vivo proliferation correlates with high response rates

• **Persistence** – longer term persistence may allow longer term disease control.

Length of persistence needed for long-term disease control is unknown.
93% CR rate for r/r ALL after CTL019

>200 patients with CLL, ALL, NHL, MM have gotten CTL019
Over 100 patients with ALL treated with CTL019 at CHOP

- 59 r/r pediatric ALL pts: 55 in CR at 1 mo (93%)
  median f/u 12 mo
- 6 went to subsequent transplant, 1 to DLI
- 6 mo RFS: 76% (95% ci 65-89%)
  12 mo RFS: 55% (95% ci 42-73%)
- No relapses past 1 year
- 18 patients in remission beyond 1 year,
  13 without further therapy
Cancer Immunotherapy Program

100 patients infused – 178 infusions

Patient Visits and Infusions per Year

- Total infusions per Year
- Total Patient Visits per Year

Visits

Year

2012 2013 2014 2015 2016 2017
Cancer Immunotherapy Program

Patients from across the US, Canada, and Mexico
Are CAR T cells effective therapy? What have we shown we can do?

- Help patients with poor disease responses before they relapse
- Get relapsed patients back into remission
- Multicenter and global trials in pediatric ALL, aiming for FDA approval Q1 2017
- Can we imagine a replacement for stem cell transplant?
- Can we move this approach into newly diagnosed high risk ALL patients?
  - Clinical trial under development
- Move to other targets and cancers
Reception

• Please come to the CHOP Hosted reception in the lobby of this building from 5-7 PM this evening, starting immediately after conclusion of today’s meeting!
Welcome

Karla Flook
People Against Childhood Cancer