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Clinical/Translational

Secretory Phospholipase A2 (sPLA2): Novel Biomarker of Acute Chest Syndrome (ACS) in Sickle Cell Disease (SCD)

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Presenting Author: Noor Alzraikat, MD (Postdoctoral Fellow)

Presentation Information: Afternoon (2:30 pm - 3:45 pm), A148-A152-A156

Abstract

BackgroundAcute chest syndrome (ACS) is a leading cause of morbidity and mortality in children with sickle cell disease (SCD), often developing during vaso-occlusive episodes (VOE) with minimal early signs. Most children present with a normal lung exam, complicating early detection. Secretory phospholipase A_2 ($sPLA_2$), an inflammatory mediator, rises before ACS onset and may predict its development and severity. We aimed to assess sPLA2 as a marker for ACS risk and severity in SCD-VOEMethodsA cross-sectional analysis of a PECARN multicenter, double-blind, phase-3 randomized controlled trial evaluating IV arginine in patients 3–21 years with SCD-VOE. sPLA2 levels at ED presentation, day 2, and discharge (DC) were measured by ELISA. Levels≥48 ng/mL were considered elevated based on SCD-specific cutoff. ACS severity was classified as mild (no oxygen/transfusion), moderate (oxygen/transfusion), or severe (BIPAP/intubation/PICU admission).Results251 subjects were analyzed (mean age 14±4 years; 53% male, 74% HbSS/Sβ^0; 75% on hydroxyurea). Mean $sPLA_2$ at ED presentation was 116±131 ng/mL; with 60% ≥48ng/mL. ACS occurred in 21%; 19 diagnosed in the ED and 34 during hospitalization. Despite normal lung exam in 85%, 71% had elevated $sPLA_2$. Patients with ACS had higher mean sPLA2 at presentation (152±146 vs 106±125 ng/mL; p=0.01) and peak levels (263±169 vs164±164 ng/mL; p<0.001), than No-ACS. The ≥48ng/mL cutoff demonstrated sensitivity=85%, specificity=33%, NPV=89% and PPV=25% for ACS detection. Mean $sPLA_2$ was highest in those with ACS and positive chest x-ray (CXR) (198±182 ng/mL), followed by those with negative initial CXR who later developed ACS (128±120 ng/mL); both significantly higher than No-ACS (106±125 ng/mL; p=0.02).Among ACS cases, 36% were mild, 46% moderate, and 17% severe, with sPLA2 increasing with severity. Mean levels in ED were 1.5-fold higher in severe vs mild cases. Peak sPLA2 levels were higher in febrile vs afebrile patients (254±19 vs 120±10 ng/mL; p<0.0001). sPLA2 levels correlated positively with heart rate (r=0.42;p<0.001), respiratory rate (r=0.14;p=0.02), white blood cells (r= 0.48;p<0.001), neutrophils (r=0.40;p<0.001), and negatively with lymphocytes (r=-0.42;p<0.001), hemoglobin/hematocrit (r=-0.26;p<0.001).Conclusion$SPLA_2$ remains a promising biomarker for ACS risk and correlates with severity in children with SCD, with potential to enhance early diagnosis and guide management.

Basic/Translational

HDAC11 Inhibition as a New Therapeutic Approach to Treating Cystic Fibrosis

Browne, Erica; Dobosh, Brian; Patel Dharmeshkumar; and Tirouvanziam, Rabindra

Presenting Author: Erica Browne, BS (PhD Student)

Presentation Information: Morning (10:30 am - 11:45 am), A148-A152-A156

Abstract

Background: Mucoobstructive lung disease in people with cystic fibrosis (CF) is driven by persistent neutrophilic inflammation, microbial tolerance, and airway remodeling. Neutrophils recruited from blood into the airway lumen play a central role in sustaining this disease cycle. Based on prior data from our group, we hypothesized that histone deacetylase 11 (HDAC11) is a key regulator of pathological programming in lung-recruited neutrophils in CF. We therefore investigated the therapeutic potential of HDAC11 inhibition using the selective small-molecule inhibitor SIS-17, and used these results to guide the development of improved HDAC11 modulators. Methods: We assessed the biochemical efficacy of SIS-17 using a high-performance liquid chromatography (HPLC) assay measuring HDAC11-dependent defatty-acylation of H3K9 and SHMT2 peptides. Functional effects were evaluated in a biomimetic human CF lung model enabling neutrophil transmigration into CF airway fluid, with readouts including neutrophil degranulation, bacterial killing of P. aeruginosa, and extracellular vesicle (EV)-associated MALAT1 expression. While SIS-17 demonstrated robust biological activity, its limited solubility motivated development of next-generation HDAC11 modulators. Accordingly, we performed an in silico screen of approximately 13 million compounds using Schrödinger's virtual screening workflow, followed by ADME prioritization with QikProp. Lead candidates were evaluated using the same HPLC assay and functional cellular models of CF lung-conditioned neutrophils. Results: SIS-17 inhibited HDAC11 activity in vitro with submicromolar IC50. In the CF biomimetic lung model, SIS-17 significantly reduced neutrophil degranulation and release of pro-tolerant MALAT1-enriched EVs while restoring bacterial killing to near-normal levels. Virtual screening identified twelve novel HDAC11 modulators, several of which demonstrated HDAC11 inhibition in biochemical assays and functional effects comparable to SIS-17 and other published HDAC11 inhibitors. Additional compounds showed potential HDAC11-activating properties.Conclusions: These findings establish HDAC11 as a central regulator of pathological neutrophil programming in CF and validate HDAC11 inhibition as a therapeutic strategy. Biological efficacy of SIS-17 provided the rationale for developing next-generation HDAC11 modulators with promising biochemical and cellular activity. HDAC11-targeted therapies represent a CFTR variant-agnostic approach that may complement existing treatments, with future studies evaluating applicability to other mucoobstructive lung diseases, including non-CF bronchiectasis.

Clinical

Improving Recognition of Acute Kidney Injury in the Neonatal Intensive Care Unit

Floyd, Chase; Hannan, Kaitlin; Gillen, Matthew; Townsend, Janae; Sim, Elaine; Shin, Stella; and Pulju, Mikki

Presenting Author: Chase Floyd, MD (Medical Resident)

Presentation Information: Afternoon (2:30 pm - 3:45 pm), A148-A152-A156

Abstract

BACKGROUND: Acute kidney injury (AKI) affects approximately 30% of infants admitted to the neonatal intensive care unit (NICU) and is associated with increased mortality, prolonged hospitalizations, and progression to chronic kidney disease. Early identification of AKI is essential for nephroprotective management and appropriate follow-up; however, fewer than 50% of neonatal AKI cases are recognized. We hypothesized that a multidisciplinary quality improvement (QI) initiative would improve recognition of AKI in NICU patients, as measured by accurate documentation on the patient problem list. METHODS: This QI initiative was conducted in two NICUs in the Atlanta metropolitan area: a level IV NICU at Arthur M. Blank Hospital and a level III NICU at Grady Memorial Hospital. The study population included NICU patients meeting neonatal-modified KDIGO criteria for AKI. Baseline AKI recognition was assessed over a six-month period. Our SMARTIE aim was to increase problem list documentation of AKI from 24% to 40% over 12 months. Interventions included presentation of baseline data to NICU faculty; a provider-focused lecture series; pharmacist-led AKI-focused discussions on rounds with serum creatinine monitoring for patients receiving nephrotoxic medications; and implementation of a resident teaching sheet with Epic SmartPhrases to promote standardized AKI documentation in provider notes. PDSA cycles were used to refine interventions. Monthly AKI recognition rates were displayed on a control chart. The balancing measure was the number of nephrology referrals that did not meet criteria for outpatient follow-up. RESULTS: At baseline, 24% of neonates meeting KDIGO criteria for AKI were correctly identified and documented on the problem list. Following sequential interventions beginning in January 2025, AKI recognition more than doubled to a sustained mean of 50% among 38 patients at Arthur M. Blank Hospital and 28 patients at Grady Memorial Hospital. Improvement was temporally associated with intervention implementation. No inappropriate nephrology referrals were identified. CONCLUSIONS: A multidisciplinary QI approach incorporating education and pharmacist engagement significantly improved recognition of AKI in the NICU. Improved identification of AKI may support timely nephroprotective management and facilitate appropriate nephrology follow-up for this high-risk population.

Basic Science

Cardiac Organoid Model of Left Ventricular Non-Compaction Cardiomyopathy

Fonoudi, Hananeh; Neupane, Achal; Negahi Shirazi, Ali; Doody, Susan; Kotamarthi, Janavi; Sapkota, Yadav ; Webster, Gregory; and Burridge, Paul W.

Presenting Author: Hananeh Fonoudi, PhD (Junior Faculty)

Presentation Information: Morning (10:30 am - 11:45 am), A148-A152-A156

Abstract

Background: Left ventricular non-compaction cardiomyopathy (LVNC) is a congenital cardiac disorder characterized by excessive trabeculation and impaired ventricular compaction, often progressing to arrhythmias, heart failure, and thromboembolic events. Pathogenic variants in MYH7 and other sarcomeric genes are strongly associated with LVNC; however, the cellular and molecular mechanisms driving this aberrant trabecular development in humans remain poorly understood. Methods: Here, we developed a multicellular cardiac organoid platform composed of human induced pluripotent stem cell–derived cardiomyocytes (hiPSC-CMs) and endocardial cells (hiPSC-EndoCs) that recapitulates early human chamber morphogenesis, including cavitation and the formation of trabecular-like projections. Results: Single-cell RNA sequencing identified four major cell populations, cardiomyocytes, endocardial cells, proliferating cardiomyocytes, and fibroblasts, and revealed that CM/EndoC interactions promote hiPSC-CM proliferation and endocardial-to-mesenchymal transition (EndoC-MT), both of which are required for trabecular maturation. Genetic validation using MYH7 knockout organoids demonstrated a marked loss of trabecular architecture, functionally confirming the identity and developmental fidelity of the structures formed in this system. To better understand the drivers of trabecular abnormalities in LVNC, we generated patient-specific hiPSC lines from three individuals with MYH7-variant LVNC, as well as from three hypertrophic cardiomyopathy (HCM) and three dilated cardiomyopathy (DCM) patients carrying pathogenic MYH7 variants, alongside three matched healthy controls. Integration of these patient-derived organoids with our developmental platform revealed an LVNC-specific phenotype: LVNC organoids exhibited hyperproliferative cardiomyocytes that failed to achieve proper trabecular specification, whereas MYH7-variant HCM and DCM organoids showed no trabecular defects. Mechanistically, LVNC endocardial cells displayed a constellation of abnormalities including elevated neuregulin signaling, altered metabolic state, impaired extracellular matrix production, and defective EndoC-MT that collectively prevent the termination cues necessary for normal trabecular maturation and compaction. Conclusion: Together, this work establishes a human-relevant framework for LVNC pathogenesis, identifying dysregulated CM/EndoC crosstalk, metabolic imbalance, and extracellular matrix defects as key drivers of disease. Our multicellular cardiac organoid system provides a powerful platform for dissecting developmental mechanisms and accelerating therapeutic discovery for congenital cardiomyopathies.

Basic/Translational

Quality and Quantity: Immune Determinants of Post-Treatment SIV Control in Early-Treated Infant Macaques

Fonseca, Jairo A; King, Alexis C; Davis, Kaleaha; Cockerham, Camryn; Liang Shan; Da Costa, Lucas; Khoei, Adrian; Ehnert, Stephanie; Wood, Jennifer; Gardner Matthew; Van Rompay, Koen; Lifson, Jeffrey; Cottrell, Mackenzie; Martins, Mauricio; and Chahroudi Ann

Presenting Author: Jairo Fonseca, MD (Junior Faculty)

Presentation Information: Morning (10:30 am - 11:45 am), A148-A152-A156

Abstract

Background: The latent viral reservoir in CD4+ T cells remains the primary barrier to an HIV cure. Latency reversal and clearance strategies combining latency reversal agents (LRAs) with immune clearance mechanisms show promise but remain unevaluated in pediatric populations. We assessed a latency reversal and clearance approach using AZD5582 as an LRA combined with AAV-delivered eCD4-IgG, an HIV entry blocker with ADCC properties, as a clearance agent in an infant non-human primate model.Methods: Twenty infant rhesus macaques were infected with SIVmac251 at week 4 of life and randomized to intervention (n=10) or control (n=10) groups. Both groups initiated ART at week 3 post-infection; intervention animals simultaneously received AAV9-delivered eCD4-IgG1. At week 40 post-infection, intervention animals received six weekly AZD5582 doses (0.2 mg/kg). Four animals per group underwent necropsy for reservoir quantification; six per group underwent analytical treatment interruption (ATI) to assess viral rebound dynamics.Results: AZD5582 successfully induced latency reversal; eight of ten intervention animals exhibited rebound viremia during dosing (p=0.0021). eCD4-Ig maintained therapeutic levels (≈10 μg/mL) for >60 weeks. Despite successful viral reactivation, most animals showed no delayed rebound at ATI. However, two intervention animals demonstrated partial virological control during ATI with significantly lower ATI viremia and lower set points than non-controllers. This control was accompanied by significant reservoir reduction in PBMCs during ATI (p=0.0226). Immunologically, controllers exhibited higher frequencies of SIV-specific CD8+ T cells pre-ATI (p=0.0357), particularly TNF+/CD107a+/Granzyme K+ triple-positive cells (p=0.03). Virological analysis revealed that controllers had lower viremia at ART initiation compared to non-controllers (p=0.0303). Importantly, after adjusting for baseline viremia at ART initiation, the magnitude of AZD5582-induced reactivation inversely correlated with both post-ATI viremia (r^2=0.694) and intact proviral DNA levels (r^2=0.639).Conclusions: AZD5582 successfully reversed latency on ART in infant macaques. Two animals exhibited partial virological control associated with enhanced SIV-specific CD8+ T cell responses. After adjusting for baseline viremia, greater AZD5582-induced reactivation correlated with improved outcomes. Results identify critical virological and immunological determinants of post-treatment control, suggesting latency reversal and clearance approaches may require patient stratification by reservoir size and immune competence, or augmentation with T cell-enhancing immunotherapies for broader efficacy.

Basic Science

Beneficial Microbe Driven Rewiring of Metabolic Pathways in the Gut and Liver

Gacasan, C. Anthony; Weinberg, Jaclyn; Naudin, Crystal; Webster, Gabrielle; Askew, Lauren; Barbian, Stefi; Jones, Dean and Jones, Rheinallt

Presenting Author: Camilo Anthony Gacasan, BS (MD/PhD Student)

Presentation Information: Morning (10:30 am - 11:45 am), A148-A152-A156

Abstract

Background: The gut–liver axis is a dynamic communication network mediated in part by gut-derived metabolites produced by both host cells and resident microbes. Alterations in gut microbiota composition and metabolic output can influence hepatic health, and supplementation with beneficial microbes may mitigate liver injury and metabolic disease.Methods: C57BL/6 mice were fed a Western-style diet and supplemented daily for 16-weeks with Lactococcus lactis subsp. cremoris (LLC; ATCC 19257, 1×10^9 CFU), Lactobacillus rhamnosus GG (LGG; ATCC 53103, 1×10^9 CFU), or vehicle (HBSS). Serum and liver metabolites were profiled using LC–HRMS and analyzed with public and in-house computational pipelines, including pathway enrichment and weighted gene correlation network analysis (WGCNA).In parallel, standard chow–fed mice were supplemented with LLC, LGG, or vehicle and subjected to an acute acetaminophen (APAP) challenge (300 mg/kg). Liver injury was assessed histologically, and hepatic gene expression quantified by RT-qPCR. To interrogate mechanism, gnotobiotic mice were mono-colonized with LLC or LGG, conventionalized, or maintained germ-free and untargeted metabolomics performed. A hybrid-targeted approach using the Bruker timsTOF platform characterized bile acid profiles in liver and fecal samples from both gnotobiotic and conventionally raised mice.Results: LLC supplementation attenuated Western diet–induced obesity and hepatic steatosis. Unbiased metabolomics revealed distinct serum and liver metabolic signatures in LLC-treated mice compared with vehicle and LGG. Pathway enrichment identified modulation of unsaturated fatty acid biosynthesis, tryptophan and riboflavin metabolism, cytochrome P450 pathways, and—consistently across models—bile acid metabolism. WGCNA highlighted bile acid–related modules as a dominant feature of LLC response.Following APAP challenge, LLC-treated mice exhibited reduced centrilobular necrosis ($P<0.05$). Hepatic expression of the bile acid–responsive transcription factor FXR and genes downstream of the NRF2 antioxidant pathway were significantly upregulated. Targeted bile acid analyses revealed pronounced remodeling of bile acid composition, with altered fecal loss and hepatic recirculation.Conclusions:LLC is a highly efficacious probiotic that establishes a favorable metabolic baseline under Western diet conditions and confers hepatoprotection against acute toxic injury. Mechanistic investigation shows LLC reprograms host metabolism through modulation of bile acid pools and gut–liver bile acid flux, highlighting microbiota-driven bile acid dynamics as a promising avenue for therapeutic and biomarker discovery.

Clinical/Translational

Transforming Behavioral and Mental Health Care through Implementation Science

Krause, Kathleen H.; Mays, Kayla; Daniel, Katherine M.; and Constantino, John

Presenting Author: Kathleen Krause, PhD, MSc (Junior Faculty)

Presentation Information: Afternoon (2:30 pm - 3:45 pm), A148-A152-A156

Abstract

Background. The need for effective children's behavioral and mental health (BMH) services has never been higher; in 2021, the American Academy of Pediatrics, American Academy of Child & Adolescent Psychiatry, and Children's Hospital Association declared a national emergency in child and adolescent mental health. To address this crisis, we describe a novel model of care being implemented at a Children's Healthcare of Atlanta (CHOA) to provide comprehensive, evidence-based BMH care.Methods. We convened an interdisciplinary BMH team to engage in intervention mapping to describe this model of care. Intervention mapping is a systematic process, based in the health promotion field, that is used to guide the design, implementation, evaluation, and adoption of an intervention (or in this case, a model of care). Following this approach, we identified the main objectives of the model of care and how we would achieve them. Results. We identified four main objectives that are needed to provide comprehensive, evidence-based BMH care; convert fragmented care to comprehensive care, focus resource allocation on higher impact evidence-based practices, consolidate accountability for the enterprise of care, and leverage existing community capacity before using internal resources. Each of these objectives was assigned a corresponding intervention strategy, a mechanism of action to operationalize the strategy within the EHR, and a behavioral theory that outlines why the specific mechanism should result in implementing an effective model of care. Conclusion. Intervention mapping served several purposes; first, it delineates the main objectives to be achieved through this model of care, second, it highlights specific mechanisms within the model of care to be evaluated (and iterated upon) for their effectiveness, both separately and as a whole, and third, it enhances the internal validity of the model of care by grounding each mechanism in a behavioral theory with its own literature that demonstrates effectiveness. Finally, intervention mapping compelled our BMH team to detail the strategies and mechanisms in granular detail so that if this model of care is proven effective, we have facilitated replication and adoption this model by other children's healthcare systems and settings.

Basic Science

Developing Circuits, Lasting Consequences: Adolescent Drug Exposure and Memory

Meadows, Mac; Gallinger, Isabel; Wolfe, Brenna; Conn, P. Jeffrey, and Luessen, Deborah

Presenting Author: Deborah Luessen, PhD (Junior Faculty)

Presentation Information: Morning (10:30 am - 11:45 am), A148-A152-A156

Abstract

Aim: Exposure to psychostimulants, such as cocaine, during adolescence produces persistent changes in the prefrontal cortex (PFC) which parallel cognitive deficits seen in adulthood. Further, adolescent exposure to psychostimulants impairs inhibitory transmission in the PFC in adulthood, suggesting that enhancing PFC inhibitory transmission may be a promising strategy to reverse drug-induced cognitive deficits. Activation of the mGlu1 subtype of metabotropic glutamate receptor increases inhibitory transmission in the PFC and working memory by selective excitation of somatostatin-expressing GABA interneurons (SST-INs). Therefore, we hypothesize that repeated exposure to cocaine during a critical developmental period in adolescence disrupts PFC inhibition via SST-INs and drives working memory impairments in adulthood which can be mitigated by activation of mGlu1.Methods: Male and female mice were injected once daily with cocaine (20 mg/kg, i.p.) for 7 days (PND 35-42). Behavioral and electrophysiological testing was conducted between 10-12 weeks of age. mGlu1 positive allosteric modulators (PAMs), SST- and PV-Ai9 tdTomato mice, whole-cell patch-clamp electrophysiology, maze- and touchscreen-based automated cognition testing. Results: We found that repeated administration of cocaine during a critical adolescent period impaired PFC SST-IN, but not parvalbumin-expressing interneuron (PV-IN), firing compared to saline-treated mice. Adolescent cocaine exposure significantly decreased the frequency of spontaneous excitatory postsynaptic currents onto SST-INs but not PV-INs. These findings were paralleled by adolescent cocaine-induced impairments in spatial working memory in adulthood. Importantly, these physiological and behavioral effects of adolescent cocaine exposure were reversed by selective mGlu1 activation. Lastly, repeated amphetamine administration during the same adolescent critical period did not result in impaired SST-IN function or spatial working memory in adulthood.Conclusions: These studies show that: 1) cocaine, but not amphetamine, exposure during an adolescent critical period induces persistent and selective deficits in PFC SST-IN function and cognition in adulthood and 2) selective activation of mGlu1 with PAMs represents a novel strategy for reversing cocaine-induced cognitive impairments.

Clinical

Windows of Opportunity: Implications of Infant Twin Research on Intervention Timing

Myers, Sarah; Narr, Peyton; Nakayama, Marina; Welburn, Aubrey; Pu, Michelle; Islam, Nailah; Davison, Todd; Reese, Derianne; Pileggi, Moira; Jones, Warren

Presenting Author: Sarah Myers, BS (Predoctoral Fellow)

Presentation Information: Afternoon (2:30 pm - 3:45 pm), A148-A152-A156

Abstract

Key Words: Genetics, Twin Background: Recent research in behavioral genetics has established 2 key findings: (1) individual genetic variation influences nearly every psychological and behavioral trait; and yet (2) genetic influence is not static but instead varies over developmental time: many psychological and behavioral traits show time-varying genetic and environmental influence. This developmental variation can be studied via the classic twin design, comparing monozygotic (identical) versus dizygotic (fraternal) twins over time. By measuring time-varying genetic and environmental influence, we can identify time periods in child development when environmental influence is greatest, and thus when therapeutic intervention for developmental delays may be most effective. We hypothesize that the extent of genetic and environmental influence on gross motor skills and expressive language will vary over developmental time. Methods: Preliminary gross motor (GM) and expressive language (EL) data were collected from twins (n=103 total, 58 MZ, 45 DZ) who completed the Mullen Scales of Early Learning (MSEL) at multiple visits between birth and 24 months (mean 3 visits per participant). Twin-twin concordance was quantified with intraclass correlation coefficients (ICCs). Results: Twin-twin concordance was statistically significant in both MZ and DZ twins at all ages for GM (rMZ=0.93-0.96, rDZ =0.78–.95) and EL (rMZ=0.77–0.82, rDZ=0.62–0.84). A significant difference in GM concordance was observed at 8–16 months (p_diff=0.012), but not at 0–8 or 16–24 months. Conversely, there was no significant difference in EL concordance at 8–16 (p_diff=0.591) or 16–24 (p_diff=0.178) months, but a trend was observed at 0–8 (p_diff=0.057) months. Conclusions:The similarity in DZ and MZ for GM development from 0–8 and 16–24 months indicates relatively strong environmental influence. The stronger MZ correlation compared to DZ for 8–16 months signifies a genetic influence and impact on the timing of major gross motor milestones around 12 months. For EL, stronger genetic influences are observed between 0–8 and 16–24 months, while environmental influence appears between 8–16 months. This pattern reflects genetic differences in early vocalizations before 6 months, yet environmental influence for first word vocalizations around 12 months. At 16–24 months, the reemergence of stronger genetic influence may relate to differences in single word vocabulary.

Translational

Engineering Chemotherapy Resistant CAR T Cells for Acute Myeloid Leukemia

Saleem, Nabil; Sullivan, Emily; Hashmi, Areeba; Fanelli, Brandon; and Raikar, Sunil

Presenting Author: Nabil Saleem, MD (Medical Fellow)

Presentation Information: Afternoon (2:30 pm - 3:45 pm), A148-A152-A156

Abstract

Background: Pediatric acute myeloid leukemia (AML) remains difficult to treat, with 5-year overall survival rates of 50-70%. CD70-directed chimeric antigen receptor (CAR) T cell products are in the clinical trial stages. While promising, CAR T therapy for AML faces unique challenges, including tumor heterogeneity, antigen escape, lack of AML-specific markers, and CAR T cell dysfunction within an immunosuppressive tumor microenvironment. To overcome these challenges, this immunotherapy can be combined with cytotoxic chemotherapy. CAR T cells can be rendered chemotherapy-resistant by knocking out deoxycytidine kinase (dCK), a key enzyme in the pyrimidine nucleoside salvage pathway that activates the commonly used AML chemotherapeutics cytarabine and fludarabine. Objective: To generate chemotherapy-resistant, AML-directed CAR T cells through dCK knockout, which will enable us to target AML using a combination immunotherapy-chemotherapy approach.Methods: We will knock out dCK in primary T cells using CRISPR/Cas9 and will study T cell function in edited and unedited cells using flow cytometry to assess for cytokine production and markers of T cell activation and exhaustion. Following knockout, we will utilize a lentiviral vector-based approach to transduce primary T cells with anti-CD70 (αCD70) CAR constructs to create dCK knockout, αCD70 CAR T cells. These cells will be incubated with AML target cells (Molm-13) with and without chemotherapy, following which a flow cytometry-based cytotoxicity assay will be used to quantify target cell death, CAR T cell persistence, and cytotoxic potential.Results: Using six dCK-specific single guide RNAs (sgRNAs) in an immortalized T cell model (Jurkat), we found two sgRNAs that achieved >95% dCK knockout efficiency. dCK-edited cells demonstrated an 18-fold increase in chemotherapy resistance compared to unedited controls, based on IC25 values calculated from chemiluminescent ATP assays. We have successfully generated both scFv-based and natural receptor-based, dCK unedited αCD70 CAR T cells that show potent cytotoxicity against AML cell lines through flow cytometry-based assays. Conclusions: dCK knockout is a feasible strategy to confer chemotherapy resistance in T cells, and αCD70 CAR T cells have potent cytotoxicity against AML in vitro. Ongoing studies using luciferase-tagged AML murine xenografts will evaluate the efficacy of this combination chemo-cellular therapy approach for AML in vivo.

Oncology & Cellular Therapy

A Small Molecule Inhibitor of EYA2 Impairs Proliferation of SIX1-expressing Pediatric Leukemias and prolongs survival leukemic mice

Aumann, Waitman; Shen, Huifen; Chen, Dongdong; Alexander, Lyndsey; Reese, Travis; Gardner, Lukas; Wang, Xiang; Zhou, Rui; Ford, Heide; and Wechsler, Daniel

Presenting Author: Waitman Aumann, MD (Junior Faculty)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 79

Abstract

Background: Overexpression of the SIX1 homeobox gene has been observed in mesenchymal and epithelial malignancies and implicated in MLL/KMT2A leukemias. With its cofactor Eyes Absent 2 (EYA2), a protein phosphatase, SIX1 transcriptionally activates developmental genes. We have demonstrated increased SIX1 expression in CALM-AF10 and other human leukemia cell lines.Methods: Wild-type and mutant SIX1 and EYA2 proteins were overexpressed in hematopoietic stem cells (HSCs) and methylcellulose culture was used to assess the impact of SIX1/EYA2 on HSC immortalization. Leukemia cell lines (Jurkat, SHI-1, and OCI-M2) were treated with a small molecule inhibitor (SMI) of the EYA2 phosphatase (LG1-34; PMID:38861151), in comparison with an inactive analog (LG1-137). Daily treatment with 100 mg/kg of LG1-34 via oral gavage commenced ten days following transplant of primary murine CALM-AF10 leukemia cells into B6 mice, or Jurkat/SHI-1 leukemia cells into NSG mice.Results: Overexpression of WT-SIX1, but not WT-EYA2, immortalizes HSCs. Overexpression of a SIX1 mutant unable to bind EYA2 does not immortalize HSCs. Combining WT-SIX1 with EYA2 mutants in the SIX1 binding region reduced HSC immortalization compared to WT-SIX1 and WT-EYA2 overexpression. LG1-34 treatment of murine CALM-AF10 and three human leukemia cell lines impaired cell proliferation in vitro, while LG1-137 had no effect having an IC50 concentration 30-90 times higher than LG1-34. Intravenous administration had higher plasma (2.8μM) and brain (2.8μM) concentrations, but shorter half-life (0.72h) compared to oral administration (1.3μM, 0.8μM, 1.63h). Treatment of LG1-34 or DMSO in mice transplanted with murine derived CALM-AF10 leukemias (n=5/group) or cell-line derived xenografts of Jurkat (T-ALL, n=10/group) and SHI-1 (AML, n=7/group) leukemias showed prolongation of survival by 3, 11, and 20 days, respectively.Conclusions: These studies demonstrate that SIX1 requires EYA2 to immortalize cells, supporting EYA2 as a potential drug target. LG1-34, an EYA2 inhibitor, impairs proliferation of several leukemias both in vitro and in vivo, and achieve effective serum levels at low doses, with IV administration requiring less drug but having a shorter half-life compared to PO dosing. Administration of LG1-34 prolonged survival of mice transplanted with both T-ALL and AMLs, suggesting that inhibition of EYA2 is a rational therapeutic approach for these leukemias.

Gastrointestinal, Hepatic & Renal

Per- and polyfluoroalkyl Substances and Temporal Changes in the Metabolome among Adolescents following Bariatric Surgery

Baumert, Brittney O.; Li, Zhenjiang; Walker, Douglas I.; Costello, Elizabeth; Ryder, Justin; Inge, Thomas; Jenkins, Todd; Sisley, Stephanie; Xanthakos, Stavra A.; Stratakis, Nikos; Valvi, Damaskini; Bartell, Scott M.; Rock, Sarah; Eckel, Sandrah P.; Aung, Max T.; McConnell, Rob; Conti, David V.; and Chatzi, Lida

Presenting Author: Brittney Baumert, PhD, MPH (Junior Faculty)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 63

Abstract

Background: Per- and polyfluoroalkyl substances (PFAS) are persistent environmental chemicals associated with dyslipidemia, insulin resistance, and liver dysfunction, yet their molecular mechanisms remain incompletely understood. Bariatric surgery induces profound metabolic remodeling in adolescents with severe obesity and provides a unique context to evaluate the relationship between exposure to PFAS and longitudinal shifts in the metabolome. Methods: We studied 186 adolescents enrolled in the Teen-Longitudinal Assessment of Bariatric Surgery (Teen-LABS) cohort (2007–2012) with fasting plasma collected at baseline (<30 days pre-surgery) and 0.5, 1-, and 3-years post-surgery. Eight PFAS congeners were quantified by LC–MS/MS (LOD=0.03 ng/mL; values <LOD imputed as LOD/2) and grouped into summed sulfonic acids and carboxylic acids. Untargeted plasma metabolomics was performed using LC–HRMS, yielding 24,375 features and 569 confirmed metabolites. Metabolite intensities were log-transformed and modeled using linear mixed-effects models with participant-specific random intercepts, including PFAS, time since surgery, and PFAS×time interaction, adjusting for age, sex, race, income, and site. A joint test evaluated PFAS main and interaction effects; multiple testing control used a PCA-derived threshold ($p<0.000085$). Pathway enrichment was conducted using mummichog.Results: Participants had mean age 17.1 years (SD=1.6); 76.3% were female and 72.0% identified as White. At baseline, PFOS (mean 6.19 ng/mL) and PFOA (3.87 ng/mL) were highest; summed sulfonic acids and carboxylic acids averaged 8.73 and 5.78 ng/mL, respectively. Significant joint tests were observed for 25–136 metabolomic features per PFAS congener. Among confirmed metabolites, PFNA was associated with increasing trajectories of medium-chain acylcarnitines (e.g., octanoyl-, lauroyl-, and decanoyl-L-carnitine), and PFDA was associated with increasing hydroxybutyrylcarnitine over time. Pathway analyses identified enrichment primarily in lipid and amino acid metabolism, including glycosphingolipid metabolism (PFOA, PFHpA) and histidine metabolism (PFOA). PFAS mixture metrics showed additional feature associations and overlapping pathway signals, suggesting dominant contributions from specific congeners.Conclusions: Plasma-PFAS measured in adolescents undergoing bariatric surgery were associated with persistent, time-dependent alterations in the plasma metabolome over a three-year period following surgery, implicating fatty acid oxidation and immune-related lipid/amino acid pathways. These findings suggest that PFAS may modulate metabolic remodeling following bariatric surgery and support integrating environmental exposures into precision approaches for pediatric metabolomic health.

Public Health, Equity & Care Delivery

Opt-Out, Step Up: Transforming Adolescent HIV Screening in Emergency Care

Bernstein, Emma; Middlebrooks Lauren; François Sandy; Brooks, Melissa N.; Byant, Jordan E.; Wynn, Bridget A.; Sadler, Annie; Cherian, Erica; Brown, Sara P.; Thompson, Sarah; Carter, Rebekah G.; DeNaples, Kelly; Kandaswamy, Swaminathan; Orenstein, Evan; Camacho-González, Andrés; Morris, Claudia R. and Griffiths, Mark A.

Presenting Author: Emma Bernstein (Undergraduate student)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 94

Abstract

The Centers for Disease Control and Prevention recommends HIV screening for all patients ≥13 years. Parts of Metro Atlanta have HIV positive rates at 8-times the national average. Adolescents are the least likely group to know their HIV status and have the lowest rate of linkage-to-care. In July 2023, Children's Healthcare of Atlanta (Children's) progressively implemented an opt-out HIV testing program in its emergency departments (ED) at all their sites for patients ≥13 years undergoing venipuncture. The aim is to assess testing rates in adolescents over the last two years.Children's electronic medical record EPIC was used to compare HIV testing volumes of patients ≥13 years old, 28 months pre and post clinical implementation. All sites received educational promotion. Results were cross-referenced to determine newly diagnosed adolescents living with HIV (ALHIV) from known positives. The data was compared using descriptive statistics.A total of 3072 patients were tested pre-implementation, 2247 (73%) females and 825 (27%) males. Eleven new ALHIV were identified; average age (± standard deviation) was 18.0 ±1.7 and the assignment at birth was male (n=8), 2 coinfected with syphilis and female(n=3). Post implementation, 6853 patients were tested: 4469 (65%) females and 2384 (35%) males. Fourteen new ALHIV were identified 5 of which were acute infections; average age was 16.2 ± 1.7 and the assignment at birth was male (n=11), 2 coinfected with syphilis, and female (n=3). This demonstrates an overall positivity rate of 0.2%; 1 in 216 boys tested positive (0.5%). At sites receiving education for 28 months, 25 and 20 months, testing increased by 78%, 92% and 193% respectively. All newly diagnosed cases were linked to care with a median of 4 days.Atlanta remains a hotspot for new HIV cases. Fourteen cases in 28 months highlight the importance of universal HIV testing of adolescents. The initiative greatly increased HIV screening among adolescents and identified some ALHIV at an early stage of infection. Future efforts include improving pediatric sexual health screening, expansion to outpatient clinics, and focusing on patients with sexually transmitted infections (STIs) for comprehensive STI preventive resources to address this public health crisis.

Neurodevelopment & Autism Interventions

Uncovering Phenotypic Convergence Across High-Risk Autism Genes Using Forebrain Assembloids

Chanda, Tanisha; Eisenberg, Carol; Heaton-Ward, Madalyn; Lee, Brandon; Simmamora, Roy; and Birey, Fikri

Presenting Author: Tanisha Chanda (Undergraduate student)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 86

Abstract

BACKGROUND: Rare and high-penetrance mutations in genes of varied functions and distinct developmental roles have been identified to confer substantial risk for autism spectrum disorder (ASD). These genes, termed "ASD genes" offer direct opportunities to understand the link between gene function and disease; however, understanding the mechanism by which mutations in genes with seemingly distinct functions throughout brain development and function converge to impart ASD risk has remained a major challenge. This lack in progress can largely be attributed to lack of access to living human tissue for functional studies. To address this issue, our lab has previously established the validity of 3D "forebrain assembloids," modelling the developing cerebral cortex using human induced pluripotent stem cells (hiPSCs). We leverage this platform, along with CRISPR-Cas9 technology, to introduce 10 ultra-rare coding variants of ASD genes into the human cortex model, and further utilize calcium imaging, microelectrode arrays (MEAs), and optogenetics to elucidate transcriptomic and functional convergence across a subset of these genes. OBJECTIVE: To identify phenotypic convergence across ten ultra-rare coding variants that have been shown to confer substantial risk to autism. DESIGN/METHODS: CRISPR/Cas9 genome editing has been used to introduce 10 rare coding variants in associated genes into hiPSCs and the successful derivation of forebrain assembloids from these hiPSC lines has been validated using bulk sequencing. We have used high-resolution 3D imaging, multi-electrode arrays and calcium imaging to interrogate the emergence of functional connectivity in these assembloids, and characterized the cell type-specific transcriptional, and gene regulatory pathways across CRISPR-edited hiPSC lines using single-cell multi-ome analysis. CONCLUSIONS: The mid-to-late gestational period of cortical development -- during which ASD genes are thought to confer susceptibility -- represent a period during which first cortical networks are assembled, and early principles of circuit formation and function are established. The neurobiological basis of how genetic perturbations in ASD genes affect the assembly of early cortical circuits in humans remains unknown, which results in the limited development of effective therapeutics. Characterization of these connectivity pathways will allow us to identify next-generation therapeutic interventions for ASD that move away from the current one gene/one drug approaches.

Infectious Disease & Immune Dysregulation

Experimental CD8 cell depletion induces viral reactivation in ART-suppressed SIV-infected rhesus macaque infants

Colvin, Alora1; Zaki Pour, Shahab1; Hamid, Riri Rizkianty1; Jeffrey Lifson2, Keele, Brandon2; Silvestri, Guido1; Chahroudi, Ann1; and Mavigner, Maud1

Presenting Author: Alora Colvin, BS

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 71

Abstract

BackgroundWhile CD8+ T cells have been implicated in controlling HIV persistence during antiretroviral therapy (ART) in adults, their activities on HIV reservoir in infants are still largely unknown. Given the distinct features of the developing immune system, we conducted a pediatric study to assess the impact of experimental CD8+ T-cell depletion on the viral reservoir in ART-treated perinatally SIV-infected rhesus macaque (RM) infants. MethodsSixteen RM infants were infected i.v. with SIVmac239M and initiated on ART 4 weeks post-infection. After >3 months of viral suppression or ≥12 months on ART, 10 RMs received a dose of the anti-CD8**α**-depleting antibody MT807R1 at 50 mg/kg, including 5 RMs that additionally received 5 weekly doses of the latency reversing agent AZD5582 at 0.2 mg/kg. Six RMs were maintained on ART only and served as controls. A comprehensive monitoring of clinical and immunovirological parameters was performed including ultra-sensitive plasma viral load measurements and immunophenotyping to evaluate latency reversal and CD8+ T-cell depletion/reconstitution. ResultsThe experimental treatment was completed in all 10 RM infants of which none experienced adverse events. The depletion of >99% of peripheral CD8+ T cells was followed by on-ART viremia at >60 copies of SIV RNA per ml of plasma in 2/5 RMs treated with MT807R1 only and 5/5 RMs treated with MT807R1+AZD5582, with a frequency of viremic episodes of 24% and 92% respectively. In the combined treatment group on-ART viremia was sustained for up to 4 weeks reaching 25,000 copies of SIV RNA per ml. While RM infants treated with MT807R1 only exhibited <1% recovery of CD8+ T cells by three weeks post-depletion, a majority of animals also treated with AZD5582 exhibited >9% at this timepoint. For both treatment groups, CD8+ T-cell reconstitution was led by cells with a memory phenotype. Conclusions[MM1] Experimental CD8+ T-cell depletion was successful and well-tolerated in infant RMs. CD8+ T-cell compartment reconstitution was slow and driven by the memory cell population. Experimental CD8+ T-cell depletion alone or with the latency reversal agent AZD5582 induced viral reactivation in ART-suppressed SIV-infected RM infants, suggesting a role for CD8+ T cells in pediatric HIV reservoir maintenance.

Cardiac Bioengineering & Platforms

Meclizine rescues cardiac function and mitochondrial ultrastructure by ATP- and glycolysis-independent mechanisms in a genetic model of mitochondrial energy dysfunction

Ghazal, Nasab; Huang, Benjamin; Shoemaker, Luke J; Faundez, Victor and Kwong, Jennifer Q

Presenting Author: Nasab Ghazal, MS (PhD Student)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 54

Abstract

Background: Mitochondrial dysfunction is a hallmark of heart failure, characterized by impaired oxidative phosphorylation (OXPHOS) and disrupted mitochondrial ultrastructure. The mitochondrial contact site and cristae organizing system (MICOS) complex plays a critical role in maintaining cristae architecture, which is essential for mitochondrial function. In the heart, loss of the mitochondrial phosphate carrier (PiC) disrupts ATP synthesis and triggers compensatory mitochondrial hyperproliferation, causing structural and functional decline. Meclizine, an FDA approved drug for vertigo, has been shown to promote metabolic adaptation, and thus, may ameliorate cardiac mitochondrial dysfunction. This study examines the effect of meclizine on cardiac function and mitochondrial structural defects in a PiC-deficient mouse model.Methods: Cardiac-specific PiC knockout mice were treated with meclizine or vehicle, and cardiac function was assessed via echocardiography. Mass spectrometry-based proteomics was used to identify molecular changes associated with meclizine treatment. Mitochondrial assays were used to assess ATP synthesis, oxygen consumption rate, and metabolic shifts.Results: Meclizine significantly improved cardiac function, increasing fractional shortening in PiC-deficient mice despite persistent mitochondrial dysfunction. Proteomics revealed that meclizine treatment causes an upregulation of subunits of the mitochondrial contact site and cristae organizing system (MICOS) complex, suggesting an impact on mitochondrial architecture. Additionally, meclizine reduced mitochondrial damage, which may restore muscle organization and enhance contractility. While mitochondrial ATP synthesis and oxygen consumption remained impaired, the observed enhanced mitochondrial ultrastructural and structural improvements point to a mechanism beyond energy production. Unexpectedly, glycolytic enzymes (PDK4 and LDH) were downregulated, with lactate levels decreasing, suggesting that meclizine can decrease lactic acidosis enhancing myocardial function without shifting to glycolysis.Conclusion: Our data suggest that meclizine's ability to restore mitochondrial ultrastructure is due in part to the upregulation of the MICOS complex and reduction of mitochondrial hyperproliferation. In this way, meclizine may function by stabilizing mitochondrial architecture to improve cardiac muscle function. These results open new doors for therapeutic strategies to improve cardiac function by targeting mitochondrial organization and offer a new pathway to mitigate mitochondrial cardiomyopathies. Future research will aim to unlock the precise mechanisms by which mitochondrial structural changes influence cardiac health and explore the broader implications of heart disease treatment.

Gastrointestinal, Hepatic & Renal

Discrepancy Between Recommended and Actual Dietary Intake For Preterm Infants Born Before 28 Weeks Gestation

Hannan, Kaitlin; He, Zhulin; Robinson, Daniel; Poindexter, Brenda; Piazza, Anthony; and McNelis, Kera

Presenting Author: Kaitlin Hannan, MD (Medical Fellow)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 65

Abstract

Background Early nutrition is critical for preterm infants with an association between adequate intake and decreased morbidity. Historical studies established early accumulated nutritional deficits of preterm infants and the correlation with growth. Since then, infants at earlier gestational ages are resuscitated more consistently, necessitating re-examination with a contemporary cohort. Objective We hypothesize that infants born 22-28 weeks gestational age (GA) do not receive goal energy and protein for a majority of the first 14 days. We hypothesize that more preterm infants, 220 to 246(group 1), accrue larger deficits than less preterm peers, 250 to 276(group 2). Lastly, we aim to correlate these deficits with growth through 36 weeks corrected GA. Design/Methods This is a retrospective study at two level III NICUs. The primary outcome was the percentage of infants who did not meet goal intake for at least 7 days. Defined goal intake included: 80kcals/kg/day with advancement to 110kcal/kg/day for energy; 3.0g/kg/day with advancement to 3.5g/kg/day for protein. We included infants born <28 weeks GA and excluded those who died before 14 days. Exact parenteral and enteral intake was collected. The difference between intake and goal was calculated to determine cumulative deficit. ANOVA compared cumulative intake between subgroups. Linear regression explored the relationship between cumulative deficits and growth Z-score trends. Results For 117 infants included, 73% did not receive goal energy intake for the majority of the first 14 days and 12% did not receive goal protein intake. Group 1 had a cumulative energy deficit of 385 kcals/kg, while group 2 had a deficit of 124 kcals/kg($p<0.001$). In contrast, protein intake for both groups yielded a net positive status: with a protein accumulation of 2 g/kg in group 1 and 8g/kg in group 2($p=0.004$). Cumulative energy deficit was compared to weight($p=0.412$), length($p=0.013$), and head circumference($p=0.203$) growth trends. The protein intake and growth relationship was not statistically significant. Conclusions Infants born <28 weeks GA did not meet goal energy intake for a majority of the first 14 days but often met or exceeded goal protein intake. Early accumulated energy deficits correlate with length growth through 36 weeks corrected GA.

Sickle Cell Disease & Transfusion Science

Acute Chest Syndrome (ACS) in Children with Sickle Cell Disease (SCD) Hospitalized for Acute Pain

Hatabah, Dunia; Ahmad, Fahd; Airewele, Gladstone; Akinsola, Bolanle; Alzraikat, Noor; Bakshi, Nitya; Brousseau, David C; Brown, Kathleen; Campbell, Andrew D; Casper, T Charles; Chang, Todd P; Chumpitazi, Corrie E; Cohen, Daniel M; Coleman, Keli D; Cruz, Andrea T; Denton, Christopher ; Ellison, Angela; Fields, Melanie E; Jensen, Hailey ; Klings, Elizabeth S; Korman, Rawan; Leibovich, Sara; Meyer, Derek; Otto, Seth; Race, Jonathan; Rees, Chris A; Remiker, Allison; Singh, Nidhi V; Thompson, Alexis A; Vichinsky,Elliott; Villella, Anthony; Wynn, Bridget ; Dampier,Carlton; Morris Claudia R

Presenting Author: Dunia Hatabah, MD (Medical Resident)

Rapid-Fire Information: Group 3 (4:00 pm – 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 99

Abstract

Background: ACS and acute pain are closely interrelated in children with SCD, and ACS commonly develops as a complication of SCD acute pain hospitalization, often prolonging and complicating it. This study aims to determine the prevalence of ACS among children hospitalized for SCD-pain, identify risk factors associated with ACS development and severity, and describe variation across institutions. Methods: Cross-sectional analysis of data from a multicenter, phase-3 trial of arginine therapy in children 3-21 hospitalized for SCD-pain at 10 pediatric EDs across the US. ACS is defined by a radiology-interpreted chest radiograph (CXR) positive for a new infiltrate. Candidate variables for the multivariable logistic regression model included demographic and clinical characteristics collected at presentation.Results: ACS occurred in 55 of 271 enrolled patients; they were significantly younger, predominantly male, and had HbSS/SBetathal0. In the ED, ACS patients had significantly more O2 desaturations <94% and a higher frequency of cough, wheeze, and chest pain than those without ACS. 83% of patients with ACS presented with a normal respiratory exam in the ED. Clinical outcomes were worse in patients with ACS vs no ACS, with significantly longer length of stay (LOS), more transfusions, O2 and BiPAP use, and PICU transfers. Of the 55 ACS, 19 were ED-diagnosed, and 36 were inpatient-diagnosed. Inpatient-diagnosed ACS had a longer LOS and a more severe ACS course vs ED-diagnosed. The mean time to diagnosis of inpatient ACS was 2.4±1.6 days. Variations in transfusion practices, antibiotic use, and clinical outcomes exist across institutions. Sex, age, ED O2 saturation <94%, hemoglobin, and chest pain were independently associated with ACS in the multivariate model, with an AUC of 0.79. Conclusion: ACS remains a significant complication in hospitalized children with SCD-pain, prolonging and complicating hospitalization. Early identification is challenging with non-specific clinical signs and symptoms, and a normal ED respiratory exam in most patients delays diagnosis. While factors identified in this study have been previously reported, ED O2 desaturation was associated with a >3.9-fold risk of ACS and was not included in prior ACS risk models. Considerable variation in ACS management exists across institutions. Further research on standardizing ACS treatment may be warranted.

Sickle Cell Disease & Transfusion Science

Transfusion Practice Variations in Children with Sickle Cell Disease

Heidari, Afshin; Alzraikat, Noor; Hatabah, Dunia; Korman, Rawan; Ahmad, Fahd; Airewele, Gladstone; Akinsola, Bolanle; Bakshi, Nitya; Brousseau, David C.; Brown, Kathleen; Campbell, Andrew D.; Casper, T. Charles; Chang, Todd P.; Chumpitazi, Corrie E.; Cohen, Daniel; Coleman, Keli D.; Cruz, Andrea T.; Denton, Christopher; Ellison, Angela; Fields, Melanie E.; Jensen, Hailey; Leibovich, Sara; Meyer, Derek; Rees, Chris A.; Remiker, Allison; Singh, Nidhi V.; Thompson, Alexis A.; Vichinsky, Elliott; Villella, Anthony; Wynn, Bridget; Dampier, Carlton; and Morris, Claudia R.

Presenting Author: Afshin Heidari, MD (Postdoctoral Fellow / Clinical Research Coordinator)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 100

Abstract

Background: Red Blood Cell (RBC) transfusion practices are not standardized in sickle cell disease (SCD). Understanding practice variations may help to standardize care and improve outcomes.Methods: A secondary analysis of a phase-3 trial that enrolled patients with SCD-vaso-occlusive pain episodes (VOE) across 10 US Childrens' Hospitals. Demographic and clinical variables were compared between children who received transfusions and those who did not using X2, Wilcoxon rank-sum, and logistic regression. Primary outcomes were the proportion transfused, indication for transfusion. Data on proportion transfused, indication for transfusion, and site-level variation in hemoglobin (Hb) thresholds and time-to-transfusion were analyzed. Results: Among 271 participants enrolled (median age;15.1 years), 63 patients (23%) were transfused. The most common indication for transfusion was ACS (n=34, 54%). Transfused children more often had a history of ACS (83.9% vs 68.9%; p=0.023) and were more likely to require oxygen (73.0% vs 18.3%; p<.0001). Of 55 patients with VOE-ACS, 34 (61.8%) received a transfusion. Site-level transfusion rates ranged from 0-36.5% for all patients with VOE and 0-100% in those with VOE-ACS. Pre-transfusion Hb averaged 6.8 g/dL and post-transfusion Hb 8.6 g/dL. Thresholds differed by site, with mean pre-transfusion Hb ranging from 6.6-7.6 g/dL. Median time-to-transfusion from admission was 2 days (IQR 1–4; range 1-5 days). In multivariable analysis, predictors of transfusion included lower presenting Hb (OR 0.59, 95% CI 0.45-0.75; p<0.001) and ED supplemental oxygen use (OR 2.60, 95% CI1.11-6.06; p=0.027).Conclusion: Transfusion practices vary widely across institutions. Low hemoglobin and oxygen requirements were strong predictors, whereas ACS was not independently predictive of transfusion. Standardization of ACS evaluation is needed, as it may be missed in patients requiring oxygen.

Public Health, Equity & Care Delivery

Evaluating How Interdisciplinary School Providers Preliminarily Deliver and Adapt Two Manualized Cognitive Behavior Therapy Programs for Autistic Students with Anxiety

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Presenting Author: Nailah Islam, BS (Research Coordinator)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 95

Abstract

Background:Implementing cognitive behavioral therapy (CBT) programs in schools may increase access to mental health services for autistic children (Reaven et al., 2024). Although research examines the effectiveness of school-based CBT, less is known about the quality of the intervention delivery. This study addressed this gap as part of a comparative effectiveness trial by evaluating Interdisciplinary School Providers' (ISPs') fidelity and adherence to Facing Your Fears- School-Based (FYF-S) and Zones of Regulation (ZOR), as well as examining how ISPs adapted these programs during implementation.Methods:Preliminary data collected from 56 ISPs across 26 school teams (36 ISPs in 16 teams trained in FYF-S and 20 ISPs in 10 teams trained in ZOR) who delivered interventions to anxious autistic students aged 8-14 years. ISPs submitted recordings from 322 FYF-S and ZOR sessions, with 132 sessions scored for fidelity by trained raters. Fidelity scoring captured both adherence to program activities and quality of delivery using a 5-point Likert scale. Scored sessions were also coded for adaptations using the Framework for Reporting Adaptations and Modifications, Expanded (FRAME).Results:Schools assigned to FYF-S completed an average of 11 of 12 lessons (M = 40.71 minutes per lesson), while schools assigned to ZOR completed an average of 15 of 18 lessons (M = 29.71 minutes). Adherence to core components was similar across interventions (FYF-S: M = 75.09%; ZOR: M = 73.55%), as was average fidelity (FYF-S: 72.21%; ZOR: 71.72%). FYF-S fidelity was higher for psychoeducation-based lessons (M = 76.41%) than for exposure-based lessons (M = 66.33%). FYF-S teams split lessons more frequently (16 times) than ZOR teams (once), while ZOR teams combined lessons more often (12 times versus once for FYF-S).Conclusions:Preliminary findings suggest comparable adherence and fidelity for FYF-S and ZOR in school settings, despite differences in session length and adaptation patterns. Findings also suggest that FYF-S may become more difficult to deliver as the lessons change from psychoeducation to exposure and may indicate that ISPs need more support delivering graded exposure within school settings. Future research will evaluate predictors of FYF-S and ZOR fidelity and will evaluate the relationship between program fidelity and student outcomes.

Infectious Disease & Immune Dysregulation

Antibodies produced after infection with WNV-1 have reduced neutralizing ability against WNV-2

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Presenting Author: Austin Leach, BS (PhD Student)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 73

Abstract

BACKGROUND: Mosquito-borne flaviviruses cause over 100 million infections each year and are among the most rapidly spreading vector-borne pathogens. West Nile virus (WNV) is the leading cause of arboviral disease in the United States and of arboviral encephalitis in the world. West Nile neuroinvasive disease is associated with high rates of mortality and long-term neurological complications, but there are no approved therapeutics or vaccines for human use. The envelope (E) protein is the surface membrane glycoprotein of WNV and is essential in virion integrity as well as mediating host cell entry through receptor binding and membrane fusion. Protective immunity against WNV is dominated by antibodies targeting the E protein, preventing viral entry by blocking either attachment to the cell surface or membrane fusion. However, there are more than 30 amino acid differences in the envelope (E) protein between lineage 1 WNV (WNV-1) and lineage 2 WNV (WNV-2) that may threaten long-term protective immunity. We hypothesized that antibodies produced following WNV-1 infection would have reduced neutralizing ability cross-lineage. OBJECTIVE: To determine the cross-neutralizing strength of antibody responses between WNV lineages. DESIGN/METHODS: We leveraged a cohort of 28 United States blood donors who tested positive for WNV-1 and returned to provide sera after 6 months. We measured the neutralization ability of their sera by measuring changes in fluorescence using a reporter virus particle (RVP) system based on a WNV-1 strain from the United States and a WNV-2 strain from Italy. RESULTS: We found that the RVPNT50 values, the reciprocal serum dilution at which the RVP infectious units are reduced by 50%, were 1472 against WNV-1 and 273 against WNV-2. 6 months after WNV-1 infection, the neutralizing ability of polyclonal sera was reduced by 5.4-fold against WNV-2 RVPs compared to WNV-1 RVPs. CONCLUSIONS: A reduction in neutralizing ability of greater than 3-fold threatens protective immunity and poses a challenge for successful vaccine development covering both circulating WNV lineages. This shows the importance of a WNV vaccine that elicits broader immunity.

Imaging & Diagnostic Tools

Language-Guided Infant Subcortical Segmentation with Pseudo-Supervision and Anatomical–Linguistic Discrimination

Liu, Ruiying; Liu, Jialu; and Wang, Yun

Presenting Author: Ruiying Liu, PhD (Postdoctoral Fellow)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am),
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 68

Abstract

BackgroundAccurate subcortical segmentation in infant brain MRI is essential for studying early neurodevelopment but remains challenging due to rapid anatomical changes, low tissue contrast, and limited high-quality manual annotations. Existing segmentation methods rely on heterogeneous labeling protocols and dataset-specific definitions, resulting in inconsistent boundaries and limited generalizability across cohorts. Although recent vision–language models introduce contextual reasoning into medical image analysis, infant neuroimaging lacks a unified anatomical framework to guide segmentation and evaluation. MethodsWe propose a language-guided framework for infant subcortical segmentation that enforces protocol-consistent anatomical reasoning under limited supervision. The framework integrates a pretrained text encoder to represent protocol-aligned anatomical descriptions—including structure shape, spatial relationships, and developmental context—with a pretrained masked autoencoder (MAE) visual backbone trained on large-scale unlabeled MRI data to address severe label scarcity in infant imaging. To further mitigate noisy or incomplete annotations, we adopt a masked pseudo-supervision strategy within a teacher–student learning paradigm, enabling progressive refinement of segmentation targets. A central component of the framework is an anatomical–linguistic discriminator that operates during training to evaluate predicted segmentations based on morphological characteristics and topology accuracy, guiding the model toward anatomically consistent outputs aligned with infant neuroanatomy.ResultsThe proposed framework was evaluated on the BCP infant cohort (1–12 months). The visual encoder is pretrained using a 3D Masked Autoencoder (MAE) on 11,948 unlabeled T1- and T2-weighted MRI scans spanning ages 1–100 years from nine publicly available datasets (e.g., ABCD and HCP), enabling robust self-supervised representation learning without manual annotations. Pseudo-supervised fine-tuning is conducted on 119 manually labeled T1-weighted subjects, drawn from ADNI, CANDI, OASIS, Colin, and BCP (12–24 months), to support protocol-aligned segmentation and cross-dataset generalization. In infant subcortical segmentation, our method achieves an average Dice Similarity Coefficient (DSC) of 75.0, outperforming the deep-learning baseline (66.3 DSC), corresponding to an absolute improvement of +8.7 DSC.ConclusionsIn this study, we combine visual representation learning with language-guided anatomical constraints for robust, data-efficient infant subcortical segmentation under heterogeneous labeling. An anatomical–linguistic discriminator enforces protocol consistency and developmental plausibility, enabling reliable subject-level comparisons. This framework provides a scalable foundation for anatomically grounded neonatal and infant segmentation.

Sickle Cell Disease & Transfusion Science

Sex and Age Effects on Complement Activity in Sickle Cell Disease

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Presenting Author: Maya Maarouf, PhD (Postdoctoral Fellow)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 102

Abstract

Introduction Vaso-occlusive episodes (VOE) and acute chest syndrome (ACS) remain the leading causes of hospitalizations and morbidity in children and young adults with sickle cell disease (SCD). Given the central role of complement activation in hemolysis and organ injury in sickle cell disease, understanding how age and sex influence complement activity may identify biologically distinct risk profiles. The objective of this study is to evaluate age- and sex- related differences in complement activation among patients with SCD during acute illness and at steady-state. Methods In this IRB-approved prospective study, we enrolled SCD patients (n=31 males, 26 females; 0-25 years old (yrs)) who were admitted for VOE or ACS at Children's Healthcare of Atlanta from 2018-2022. Blood samples were collected within 48 hours of admission (acute) and at clinic follow-up (steady-state) ≥4 weeks later. Levels of proximal (Bb, C4, C4a, C4d), common (C3, C3a), and terminal (C5, C5a, C5b-9) complement pathway markers were measured using ELISA. Samples from race-matched pediatric healthy controls (n=17, 0-16 yrs) were also analyzed. Statistical analyses used parametric or non-parametric tests as appropriate, with significance defined as $p<0.05$. Results At steady-state, no significant sex-related differences were observed across proximal, common, or terminal complement markers in any age group. During acute VOE/ACS admissions, males and females in the <12 and 12–18 year groups demonstrated comparable complement levels. In contrast, females older than 18 years exhibited higher levels of C3 (mean=62.47 vs 49.57 mg/dL, p=0.0107) and C5b-9 (mean=256.5 vs 170.1 ng/mL, p=0.0235) compared with age-matched males during acute episodes. No sex-based differences were identified among race-matched pediatric healthy controls. Overall, complement activation profiles were similar between sexes across pediatric and adolescent patients, with differences noted only in adult females during acute illness. Conclusions Overall, complement profiles were largely similar between males and females, both during VOE/ACS and at steady-state. The observed elevation of C3 and C5b-9 in adult females during acute episodes suggests a potential age- and sex-dependent divergence in complement activity. Although limited by sample size, these findings warrant further investigation to clarify the clinical and therapeutic implications of complement dysregulation in adult females with SCD.

Infectious Disease & Immune Dysregulation

Human Cortical Organoid as a Model for West Nile Virus Infection

Malakar, Shilu; Suresh, Supriya Wariyar; Andersen, Jimena and Suthar, Mehul S.

Presenting Author: Shilu Malakar, PhD (Postdoctoral Fellow)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 75

Abstract

The emergence of neurotropic arboviruses presents a significant global health threat, demanding biologically relevant models to study viral pathogenesis and evaluate countermeasures. West Nile virus (WNV) is a leading cause of Orthoflavivirus-induced encephalitis in the United States. WNV infects both adults and children, and understanding age-specific risks may help improve early diagnosis, develop safe treatments and vaccines, and prevent long-term neurological complications. Although substantial progress has been made in elucidating WNV biology and pathogenesis, effective therapeutic agents and prophylactic vaccines for human application remain to be developed. WNV infects the central nervous system (CNS), yet its precise neurotropic mechanisms remain poorly understood due to limitations in current model systems. Leveraging human induced pluripotent stem cell (hiPSC) technology combined with three-dimensional (3D) culture methods, we gain a unique opportunity to model previously inaccessible human neurobiology. We developed a novel human cortical organoid model to investigate WNV neurotropism and host immune response. These 3D cellular structures closely recapitulate the cytoarchitecture and cellular complexity early human cerebral cortex. Cortical organoids were infected with WNV across a dose range (105, 106, and 107 PFU), and infection progression was monitored for three days. Preliminary results confirmed a successful and sustained viral infection, shown by a dose- and time-dependent increase in viral load quantified by real-time RT-qPCR and standard virus titration assays. Furthermore, transcriptional analysis confirmed the robust innate and antiviral response within the organoids, including significant upregulation of pattern recognition receptors, interferons, and interferon-stimulated genes. These findings establish human cortical organoids as a valuable model for studying the neurotropic mechanisms of WNV. This platform provides significant advantages over traditional cell culture and non-human animal models, offering a physiologically relevant system for investigating human viral infections and host immune responses in the CNS.

Infectious Disease & Immune Dysregulation

22q11.2 deletion syndrome is characterized by an inflammatory plasma profile and CD4 T cells skewing towards Th1/Th17

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Presenting Author: Christina Michalski, PhD (Postdoctoral Fellow)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 76

Abstract

Background: Chromosome 22q11.2 deletion syndrome (22qDS) is a rare genetic disorder caused by microdeletions of 1-3 MB on chromosome 22. The clinical phenotype is highly variable but typically impacts the cardiovascular, immune, and nervous systems. Children with 22qDS are at high risk for neurodevelopmental disorders including autism spectrum disorder, attention-deficit/hyperactivity disorder, anxiety, schizophrenia, and intellectual disability. Previous studies have suggested a role for inflammation in neurodevelopmental disorders, including 22qDS. However, the immune milieu in 22qDS remains incompletely characterized. Methods: We collected plasma and peripheral blood mononuclear cells (PBMCs) from individuals with 22qDS and age- and sex-matched healthy control (HC) donors (n=34 and n=33, respectively). Plasma proteins, with a particular focus on cytokines and chemokines, were quantified using the Mesoscale Discovery platform. Single-cell RNA sequencing of the PBMCs was performed using Seq-well technology by Parse Biosciences. Results: Plasma levels of pro-inflammatory cytokines such as IL-1**β**, IL-6, and IL-17A were significantly elevated in 22qDS compared to HC. Interestingly, we also observed a significant correlation of plasma cytokine levels with schizophrenia-associated symptoms (Structured Interview for Psychosis-risk Syndromes scores), implying a link between systemic inflammation and neuropsychiatric outcomes in 22qDS. Using scRNA-seq, we confirmed that donors with 22qDS have reduced expression of genes in the 22q11.2 deletion across all major PBMC subsets. 22qDS PBMCs had decreased frequency of naïve CD4 T cells, while the frequency of memory CD4 T cells with Th1/17 phenotype was increased in 22qDS samples and correlated with plasma IL-17A levels. Differential gene expression analysis followed by gene set enrichment analysis further demonstrated that pathways related to T cell activation were upregulated in 22qDS CD4 T cells. Conversely, inflammation-related genes were downregulated in monocytes and DCs, suggesting cell-type specific effects of 22qDS. Conclusion: Together, our results suggest that in 22qDS, CD4 T cells are more differentiated and skewed towards a pro-inflammatory Th1/Th17 phenotype, likely driving the high levels of inflammatory plasma cytokines. Previous studies have linked IL-17A to neuropsychiatric symptoms in idiopathic schizophrenia, autism spectrum disorder, and major depression. While further mechanistic studies are essential, our results suggest that anti-inflammatory therapeutics may be beneficial in 22qDS.

Sickle Cell Disease & Transfusion Science

Storage-induced microerythrocyte content in red blood cell (RBC) units transfused to patients with sickle cell disease

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Presenting Author: Zahra Naseh, MD (Postdoctoral Fellow)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm),
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 103

Abstract

Background: Patients with sickle cell disease (SCD) often require frequent and life-saving red blood cell (RBC) transfusions. The response to each transfusion is variable, with little known about the factors influencing transfused RBC lifespan. During cold storage, donor RBCs experience molecular and morphologic alterations, known as the storage lesion, which may impact RBC survival and compromise transfusion efficacy. We have previously evaluated morphologic changes of stored RBCs and the development of storage-induced microerythrocytes (SMEs). The current study aims to define the percentage of SMEs throughout storage in RBC units that were transfused to patients with SCD. Methods: Segments of tubing from RBC units that were ≤23 days old when transfused to patients with SCD for chronic erythrocytapheresis were collected from the bedside. RBC concentrates were stained with carboxy-fluorescein-succinimidyl-ester (CFSE) fluorescent dye and incubated at 37°C for 48 hours. Flow cytometry was utilized to measure CFSE concentration and to identify and quantify SMEs. RBCs were tested once weekly until the date of expiration (42 days). Imaging flow technology and electron microscopy were used to visualize CFSE-high and CFSE-low RBC populations and confirm SME morphologic changes. Results: We tested 29 RBC units with a mean age of 20 days on the first day of testing. The percentage of SMEs in each RBC unit increased at each timepoint tested. Overall correlation between RBC unit age and SME percentage was $r=0.65$, $p<0.0001$. Mean increases in SME percentage of each RBC segment were: 2.1% (0.2 – 8. 6%) increase from week 1-2; 4.7% (1.0 – 13.7%) increase from week 2-3; 7.6% (2.0 – 18.4%) increase from week 3-4. Imaging flow and electron microscopy confirmed SME morphologic changes in high-CFSE RBC populations. Conclusion: The correlation SME concentration to the age of the RBC product underscores the importance of studying RBC quality during storage to ensure optimal transfusion outcomes. Storage changes may have a negative impact on donor RBC quality and hence transfusion effectiveness, particularly in patients with SCD. Future studies will evaluate the correlation of SMEs in RBC units transfused to direct measurements of transfused RBC in patients with SCD.

Public Health, Equity & Care Delivery

Utilization of a Therapy Referral Process for Adverse Childhood Experiences (ACEs) in Pediatric Emergency Department (PED) Patients

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Presenting Author: Karli Okeson, DO (Junior Faculty)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 96

Abstract

Background:Adverse Childhood Experiences (ACEs) are common among pediatric emergency department (PED) patients. ACEs screening and warm therapy referrals may be a strategy to address ACEs in the PED. This study aimed to determine behavioral mental health (BMH) therapy resource utilization between in-person versus telehealth referrals and compare parental versus child-reported ACEs and Positive Childhood Experiences (PCEs) in PED patients.Methods:English-speaking PED patients 12-17 years and their caregivers completed surveys assessing the patient's ACEs, PCEs, therapy referral preferences, and perceived treatment barriers. Participants with ACEs ≥2 were contacted by a BMH specialist at least twice to arrange an in-person or virtual therapy referral via a randomized controlled protocol and subsequently asked if an appointment was attended. Patients already in therapy were excluded.Frequencies, percentages, medians and interquartile ranges are reported. Parent and child ACEs/PCEs comparisons used the McNemar's test, Wilcoxon sign rank test, and Krippendorff's alpha. Age and ACEs/PCEs association were evaluated using linear correlations.Results:Of 61 participants, 28 (45.9%) had ≥2 ACEs. Most were female (59%), Black (75.4%), and Non-Hispanic (96.7%).There were 7/28 (25%) families who responded to the BMH specialist for therapy referrals; only 1 (3.6%) confirmed attending an in-person therapy appointment. The most reported perceived barrier to therapy was work schedule conflicts (32.1%). Most caregivers (57.1%) had no preference for appointment type; among those with a preference, most preferred telehealth (39.2%).Caregivers and patients reported similar median scores for ACEs (1) and PCEs (7). Age-by-respondent interaction was significant in ACEs (p=0.02) and PCEs (p=<0.001). Age was significantly positively correlated with caregiver-reported PCEs (r=0.39 [CI:0.16-0.56]) but not child-reported PCEs (r =-0.10 [CI:-0.34-0.18]), caregiver-reported ACEs (r =-0.07 [CI:-0.34-0.23]) or child-reported ACEs (r =0.22 [CI:-0.07-0.50]).Conclusion:PED-based ACEs screening with warm therapy referrals showed minimal benefit as few patients pursued BMH resources regardless of in-person vs virtual format. Work scheduling conflict was the highest perceived barrier to therapy. Although median ACEs and PCEs scores were similar, parent and child reports often differed, emphasizing the need for both perspectives. Further research is needed to determine effective processes for screening and connecting patients to care.

Imaging & Diagnostic Tools

Low-Dose Chest CT Accurately Detects Airway Foreign Bodies in Children: A Retrospective Diagnostic Accuracy Study

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Presenting Author: Carlotta Onnis, MD (Medical Fellow)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD

Poster Number: 69

Abstract

Background Foreign body (FB) aspiration is a common medical emergency in children, which can be fatal. Prompt diagnosis is crucial to avoid complication. Bronchoscopy represents the gold standard for diagnosis; however, false negatives can lead to unnecessary invasive testing and exposes children to procedural risks. While lateral decubitus radiographs can assess for air trapping, this exam has low sensitivity for detecting airway foreign bodies. Thus, there is a need for a sensitive and specific diagnostic imaging test in the detection of airway FB. We developed and implemented a FB chest CT protocol that is characterized by low-dose relative to a standard chest CT and no intravenous contrast. We hypothesize that low-dose chest CT is a sensitive and specific modality for detection of airway FB in the pediatric population that may ultimately decrease the negative bronchoscopy rate Methods The retrospective study was performed at a quaternary care children's healthcare system in a major metropolitan city. There were 56 patients included, aged 0-18 years, who underwent low-dose chest CT and bronchoscopy for suspected FBA, between June 2022 and March 2025. Sensitivity, specificity, positive predictive value (PPV), negative predictive value (NPV), and accuracy were used to evaluate the performance of low-dose chest CT compared to the gold standard. Radiation dose was an additional metric evaluated. A 95% confidence interval was calculated. Results A total of 56 patients were included (mean age 4.3 ± 4.3, 51.8% male). Low-dose CT was negative in 46/56 cases, with one false negative (1/46), proven positive by bronchoscopy. CT yielded positive results in 10/56 cases, which were all true positives. We observed a sensitivity of 90.9% (95% CI: 58.7-99.8), specificity of 100% (95% CI: 92.1-100), PPV of 100% (95% CI: 69.2-100), NPV of 97.8% (95% CI: 88.5-99.9), and accuracy of 98.2% (95% CI: 90.4-100). The median radiation dose with interquartile range for the exam was 0.41 [0.27 – 0.60] mSv. Conclusions Low-dose chest CT can accurately identify FB aspiration in children. Given its excellent specificity, NPV, and low radiation exposure, low-dose chest CT can safely rule out airway FB and may ultimately reduce unnecessary bronchoscopies in suspected airway FB evaluation.

Neurodevelopment & Autism Interventions

Characterizing the Effects of Sociodemographic Indicators on Caregiver Interactive Behaviors in Dyads with Infants Across the Autism Spectrum

Ravichander, Aanya; Shultz, Sarah; and Ford, Aiden

Presenting Author: Aiden Ford, PhD on behalf of Aanya Ravichander (Undergraduate student)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 90

Abstract

Background: Caregivers adjust their interactive behaviors based on their perceptions of their infants' social ability, and prior research suggests that household income and emergent social differences related to later autism outcome may shape this process. This study aimed to evaluate the predictive effect of three socioeconomic indices - maternal education, income-to-needs ratio, and Child Opportunity Index - on the use of caregiver greeting, an infant-directed signaling behavior, in infant-caregiver dyads with neurotypically-developing infants (NT group) or infants with an elevated likelihood of an autism diagnosis who did or did not receive an autism diagnosis (AUT or el-nAUT, respectively). Methods Dyads (NT=90, AUT=45, ELNASD=49) completed screen-mediated interactions at up to six timepoints from 0-6 months of age. Greeting behavior (simultaneous widening of the eyes, eyebrows, and mouth) was manually coded at the beginning of each interaction. Maternal education and household income were self-reported at enrollment. Income-to-needs ratio, estimating proximity to poverty threshold, was derived from household income and family size. Child Opportunity Index, a composite measure of neighborhood resource access, was derived from self-reported address. Generalized Additive Models (GAMs) evaluated the predictive effect of socioeconomic indices on greeting behavior over time. Results AUT dyads had significantly lower income-to-needs ratios than NT and el-nAUT dyads ($p<0.05$), tended towards lower Child Opportunity Index values than NT and el-nAUT dyads ($p<0.1$), and had lower maternal education levels than NT dyads ($p<0.05$). GAM models evaluating how these metrics predict greeting across all dyads showed greeting is best predicted by incorporating income-to-needs ratio, Child Opportunity Index, and maternal education as fixed effects. Post-hoc evaluation showed that income-to-needs significantly predicted greeting behavior ($z=2.11$, $p=0.035$), with higher income-to-needs predicting greater likelihood of greeting. Conclusion The best-fitting model predicting greeting likelihood includes Child Opportunity Index, income-to-needs ratio, and maternal education, highlighting the importance of including multiple socioeconomic indicators in analyses of early social interaction. Socioeconomic differences were evident between neurotypical and autism dyads and within elevated-likelihood groups, underscoring the importance of evaluating socioeconomic differences in early trajectories of social learning. Future directions will assess if the predictive relationship between income-to-needs and greeting behavior differs by diagnostic outcomes.

Cardiac Bioengineering & Platforms

A model multi-systems approach for understanding the role of the PIX pathway in cardiac muscle and dilated cardiomyopathy

Shoemaker, Luke; Ghazal, Nasab; Kalan, Tiara; Qadota, Hiroshi; Kwong, Jennifer, Kwong; Benian, Guy

Presenting Author: Luke Shoemaker, BS (PhD Student)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 57

Abstract

Cardiomyopathies represent a substantial burden on the global healthcare system. While many cases are associated with mutations in genes encoding mostly sarcomeric proteins, in many other cases the genes involved are unknown. Thus, there remains a significant need for the discovery of new genetic factors. Our laboratory has reported that loss-of-function mutations in the C. elegans gene pix-1 result in a loss of assembly of integrin associated complexes (IAC) in striated muscle. PIX-1 is a Rac GEF that activates Rac, and thereby activates its protein kinase PAK, although the substrates for this kinase are unknown in any muscle tissue. The PIX pathway is evolutionarily conserved. In mammals, the PIX-1 ortholog is **β**-PIX, but its role in mammalian muscle remains poorly understood. Because mutations in some IAC components result in cardiomyopathy, we postulated that the gene for **β**-PIX might also be involved in cardiac muscle regulation and disease. First, we localized **β**-PIX in the mouse heart and found that it localizes to intercalated disks, costameres, and Z-disks in cardiomyocytes, structures analogous to those in nematode muscle where PIX-1 is located (the bases of M-lines and dense bodies, and muscle cell boundaries). We made a cardiomyocyte-specific knockout of **β**-PIX and found that the mice develop dilated cardiomyopathy by 8 months of age and die between 8 and 11 months of age. In the **β**-PIX KO at 8 months of age, intercalated disks have an increased amplitude, measured as a higher ratio of contour length to chord length. Although we found no differences in the levels of various IAC proteins, we found that even at 2 months of age, before cardiomyopathy is evident, there is a loss of integrin-linked kinase (ILK) at costameres. Additionally, Kindlin-2, another known IAC component, forms aggregates at 8 months of age. Together, these results provide insight into how loss of **β**-PIX perturbs integrin adhesion signaling and contributes to cardiomyopathy development.

Cardiac Bioengineering & Platforms

Photo-Driven Tissue Editing Creates In Vitro Microstructural Arrhythmia Substrates in Pediatric Cardiac Tissue

Son, Young Hoon, Won, Jihee; and Park, Sung Jin

Presenting Author: Young Hoon Son, PhD (Postdoctoral Fellow)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 58

Abstract

BACKGROUND: Pediatric ventricular arrhythmias often arise from small structural abnormalities in cardiac tissue, such as localized cell loss, excess fibroblasts, and tissue discontinuities that interfere with normal electrical conduction. These microstructural features play a critical role in arrhythmia initiation and maintenance but are difficult to recreate using additive tissue engineering approaches alone. As a result, mechanistic studies and therapeutic testing in pediatric relevant in vitro models remain limited. The objective of this study was to develop an in vitro method to precisely edit cardiac tissue structure to model pediatric arrhythmia substrates. METHODS: We performed an in vitro tissue engineering study using neonatal rat ventricular myocytes (NRVMs) and neonatal rat cardiac fibroblasts (NRCFs), and human induced pluripotent stem cell derived cardiomyocytes (hiPSC-CMs) cultured as confluent cardiac tissues. Photo-driven tissue editing was performed by engaging endogenous cellular remodeling mechanisms to allow precise editing of tissue structure under low intensity patterned light. Micro scale tissue edits (50–100 μm) were generated using microfabricated photomasks or a digital micromirror device to create defined spatial patterns within living cardiac tissues. After tissue editing, cell viability was assessed to confirm cytocompatibility. Cardiac gene expression was analyzed to verify preservation of cardiac identity, and cell migration into edited regions was monitored over time to evaluate tissue remodeling. Macrophages were added to assess clearance of edited cells and stabilization of tissue boundaries. RESULTS: Photo-driven tissue editing produced well-defined and reproducible micro scale cell free regions while maintaining high viability in surrounding tissue. Cardiac gene expression remained stable after tissue editing, indicating that cardiac cell identity was preserved. Over time, neighboring cardiac cells migrated into the edited regions, demonstrating maintained tissue remodeling capacity. Macrophage mediated clearance efficiently removed edited cells, resulting in stable micro-structural boundaries that resemble scar-like conduction barriers observed in pediatric ventricular arrhythmia substrates. CONCLUSIONS: Photo-driven tissue editing enables precise in vitro remodeling of pediatric cardiac tissue structure to recreate micro-structural features relevant to ventricular arrhythmias. This approach provides a disease-focused in vitro model for studying pediatric arrhythmia mechanisms and for evaluating therapeutic strategies in cardiac tissues with defined structural architecture.

Cardiac Bioengineering & Platforms

A Vascularized 3D-Bioprinted Myocardial Platform for Recapitulating Pediatric Congenital Heart Disease

Sridhar, Vani; Serpooshan, Vahid; Kaza, Pranitha; and Fonoudi, Hananeh

Presenting Author: Vani Sridhar, MS (PhD Student)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 59

Abstract

BACKGROUND: Congenital heart diseases (CHDs) are complex structural and functional disorders that remain the leading cause of birth defect-related mortality. Current understanding of CHD development is hindered by animal models that fail to replicate human-specific cardiac physiology and patient-specific genetics. We hypothesize that a vascularized, 3D-bioprinted human myocardium could serve as a tunable, biomimetic platform to model CHD progression. OBJECTIVE: To engineer a high-fidelity, 3D-bioprinted human myocardial platform for the study of pediatric CHDs. METHOD: Cuboidal myocardial analogues with bifurcated vascular channels were designed via CAD and fabricated using high-resolution stereolithography with a custom bioink. The platform incorporates hiPSC-derived cardiomyocyte (CM) spheroids and ECM-mimicking hydrogel to recreate the myocardium and endothelial cells (ECs) in relevant physiological ratios within the vasculature. Constructs were cultured in custom perfusion chambers under physiological flow conditions for 14 days. Structural fidelity, mechanical stiffness, cell viability, and contractile kinetics were quantified. Computational fluid dynamics (CFD) analysis was performed on the geometry to assess flow velocity, wall shear stress (WSS), and mass transport. Sample sizes of n=6 and Student's t-tests were used to assess statistical significance ($P < 0.05$). RESULTS:Fidelity studies confirmed >90% structural stability for bioprinted bifurcated vascular geometries. Microindentation confirmed an elastic modulus of ~4 kPa for the interstitial ECM hydrogel (optimal for CMs) and ~10 kPa for the channel lumen (suitable for EC function). Immunostaining confirmed robust lumenal endothelialization and uniform distribution of viable hiPSC-CM spheroids. Notably, CM spheroids exhibited contractile forces exceeding 70% of those of 2D monocultures. CFD analysis visualized WSS, oxygen, and nutrient transport across the construct, validating the physiological relevance of the model. CONCLUSIONS:This platform enables the creation of personalized models using patient-specific hiPSCs to study the mechanisms underlying rare pediatric genetic diseases. By achieving >70% of 2D contractile force and maintaining high-fidelity bifurcated vascular geometries, this system provides a biomimetic environment suitable for longitudinal studies of CHD. Most importantly, integrating patient-specific hiPSCs enables investigation of the mechanistic drivers of rare pediatric genetic diseases and supports personalized drug screening.

Oncology & Cellular Therapy

Medulloblastoma Circulating Tumor Cells Differ from Primary Brain Tumor Cells

Stewart, Martha; Chien, Frank; and MacDonald, Tobey

Presenting Author: Martha Stewart, MD (Medical Fellow)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 81

Abstract

Background: Medulloblastoma is the most common malignancy of the childhood central nervous system (CNS) and is a leading contributor to cancer-related mortality in pediatrics. Currently, there is no test for minimal residual disease (MRD) Challenging previously held beliefs that cancer spreads by direct leptomeningeal shedding, preclinical evidence now demonstrates a hematogenous route for metastasis, highlighting the opportunity for liquid biopsy. We developed a label-free approach for collecting circulating tumor cell clusters (CTCCs). We hypothesize that captured CTCCs represent a sub-clonal population of cells distinct within the primary tumor with stem-like properties that drive treatment resistance and relapse, providing key, non-invasive insights into tumor biology. Objective: To compare and contrast the genetic, metabolic, and drug-responsiveness of medulloblastoma circulating tumor cell clusters and corresponding primary brain tumor cells. Methods: Primary brain tumor (BT52) and corresponding CTCCs (BT52 CTCCs) collected from the serum of a patient with group 3 medulloblastoma were cultured in suspension. Cells were sent for single cell DNA and RNA sequencing. Baseline metabolic characteristics including oxygen consumption rate (OCR) and extracellular acidification rate (ECAR) were assessed using the Seahorse Mitochondrial Stress Test. Drug sensitivity using high throughput screening with over 2000 drugs was performed. Further comparisons including cell-cycle analysis and proteomic data pre- and post-treatment are planned. Results: Preliminary single cell sequencing confirmed matching DNA and RNA copy number variations for BT52 and BT52 CTCCs. BT52 CTCCs had a higher maximum respiratory capacity than BT52s as measured by OCR (36.68 vs 28.22 pmol/min, p 0.0072) but no difference in basal respiration (34.33 vs 31.88 pmol/min, p 0.0148). BT52 cells had a higher ECAR than BT52 CTCCs through all phases of the mitochondrial stress test (16.93 vs 8.52 mpH/min, $p<0.0001$). BT52 and BT52 CTCCs did not show similar inhibition to all drugs tested. Conclusions: Preliminary data suggests BT52 and BT52 CTCCs share similar DNA and RNA profiles, however BT52 CTCCs have a higher maximal respiratory capacity, rely less on glycolysis, and have different drug sensitivity than BT52 primary tumor cells. Patient-derived CTCCs can be used to non-invasively evaluate medulloblastoma characteristics to further elucidate response and mechanisms of resistance.

Oncology & Cellular Therapy

The function of LBX1 in Wilms tumor

Sijia Tang and Andrew L. Hong

Presenting Author: Sijia Tang, PhD (Postdoctoral Fellow)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 82

Abstract

Wilms tumor (WT) is the most common pediatric kidney cancer, and relapsed WT remains a leading cause of cancer-related mortality in children. When WT recurs, the intensive therapies required often result in substantial morbidity and treatment-related complications. Therefore, there is an urgent need to develop therapeutic strategies that maintain anti-tumor efficacy while minimizing long-term toxicities. Progress toward this goal has been limited by an incomplete understanding of the molecular mechanisms that sustain WT development and maintenance.Our data from a manuscript under revision reveal an unexpected role of the RAS/MAPK-SIX2 axis in WT biology. Furthermore, inhibition of MEK1/2 with trametinib decreases WT cell viability by more than 50% and suppresses SIX2 expression. However, the persistence of viable cells suggests that additional pathways are required to sustain WT survival. Therefore, we analyzed RNA sequencing (RNA-seq) data to compare WT samples with normal kidney tissues and matched cell lines. Multiple homeobox genes are consistently upregulated, with ladybird homeobox 1 (LBX1) identified as a novel and significantly upregulated candidate. CRISPR-Cas9-mediated deletion of LBX1 significantly reduced WT cell proliferation, suggesting that LBX1 functions as a critical regulator of tumor maintenance. Unlike SIX2, LBX1 expression persists despite MEK1/2 inhibition, suggesting a MEK-independent mechanism that sustains tumor identity and partial resistance to therapy. To define the molecular programs regulated by LBX1, transcriptomic profiling by RNA-seq revealed widespread changes in gene expression associated with developmental and signalling pathways relevant to nephron progenitor identity. Ongoing chromatin accessibility and histone modification profiling aim to define the epigenetic mechanisms through which LBX1 regulates these transcriptional programs.Collectively, these preliminary findings support that LBX1 may contributes to the maintenance of developmental transcriptional states in WT and influences therapeutic response. Future studies will focus on defining LBX1-dependent regulatory networks and evaluating whether targeting LBX1-associated pathways may enhance treatment efficacy while minimizing toxicity in WT.

Sickle Cell Disease & Transfusion Science

As a participant, you are an expert: Experiences of an integrative treatment program for teens living with sickle cell disease and chronic pain and their caregivers

Mooney, Jan T.; Brasher, Susan N.; Upadhyay, Una; Adkins, Taylor R.; Williams, Justin; Nuñez, Anjanette; Dampier, Carlton D.; Crosby, Lori E.; Kashikar-Zuck, Susmita; Thomas, Staci; Lang, Amy; Murphy, Bridget; Akintobi, Tinu; Batts, Keenan; Kurzhals, Aschli; Quinn, Charles T.; Myer, Gregory D.; Bakshi, Nitya; Kesar, Trisha and Sil, Soumitri

Presenting Author: Unnati Upadhyay, PsyD (Postdoctoral Fellow)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 104

Abstract

IntroductionChronic pain affects approximately one-fifth of youth with sickle cell disease (SCD). Although best-practice guidelines recommend integrative, multicomponent care, there is a critical, unmet need for evidence-based interdisciplinary approaches for chronic SCD pain. I-STRONG was co-developed with teens with chronic SCD pain and their caregivers, community advisory boards, and interdisciplinary experts, leveraging an existing group-based program for chronic widespread pain combining cognitive-behavioral therapy (CBT) and neuromuscular exercise training. In its pilot study, I-STRONG demonstrated quantitative feasibility (e.g., adherence), acceptability (e.g., satisfaction ratings), safety (i.e., minimal adverse events), and most participants reported overall pain improvements. The aim of the present qualitative work was to further understand patient and caregiver experiences of I-STRONG, to guide iterative program improvements. MethodsAdolescents from two sites in the United States, aged 12-18 (any SCD genotype, medium/high risk on Pediatric Pain Screening Tool), and their caregivers participated in the I-STRONG pilot study. Semi-structured interviews guided by the RE-AIM framework were analyzed via hybrid deductive-inductive framework approach. ResultsAdolescents (n = 11; 16 ± 1.60 years) were Black/African American with 92% Medicaid-covered. Caregivers (n = 6; 41 ± 4.99 years) were Black/African American and 92% mothers. Preliminary qualitative analyses supported feasibility and acceptability of the program. Teens and caregivers felt connected and understood in the group, though noted the time commitment of attendance. Participants appreciated individualization to support participation across a range of abilities. Teens improved daily functioning and tolerance for physical activity, also integrating I-STRONG skills into their pain management strategies after the program. ConclusionsAligned with and expanding on the findings from the pilot study quantitative indices, adolescents and caregivers indicated that I-STRONG is highly feasible, well-tolerated, acceptable, and responsive to individual needs. Next steps include testing I-STRONG in a multi-site randomized clinical trial to further examine its unique impact on pain and functioning.

Cardiac Bioengineering & Platforms

Source–Sink Microtissues Orchestrate Cardiac Rhythm via Tuned Ion-Channel Balance in 2D and 3D Constructs

Won, Jihee; Son, Young Hoon; Atinksy, Audrey; and Park, Sung Jin

Presenting Author: Jihee Won, PhD (Postdoctoral Fellow)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
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Poster Number: 60

Abstract

BACKGROUNG: Pediatric arrhythmias are serious cardiac disorders caused by unstable electrical activity and can lead to severe clinical outcomes. During heart development, immature electrical coupling and weak pacemaker control allow multiple initiation sites to emerge, increasing susceptibility to rhythm instability and tachyarrhythmias. Pacemaker activity depends on the balance between depolarizing and stabilizing ionic currents. But how this balance regulates rhythm across different cardiac tissue architectures remains poorly understood. The objective of this study was to develop an in vitro cardiac tissue model to investigate how pacemaker activity can be enabled and stabilized by tuning ion channel balance and tissue structure. METHODS: Cardiac pacemaker tissues were engineered using neonatal rat ventricular myocytes (NRVMs) and human induced pluripotent stem cell derived atrial like cardiomyocytes (iPSC-CM). Pacemaker activity was induced by overexpressing hyperpolarization, activated cyclic nucleotide gated channel 4 and assembling cells into compact spheroids. Inward rectifier potassium current was tuned through Kir2 channel modulation to adjust the electrical balance between the pacemaker source and surrounding myocardium. Pacemaker spheroids were integrated into two dimensional and three dimensional microengineered cardiac tissues. Electrical activity was assessed by optical mapping to evaluate initiation sites, rhythm regularity, beat to beat variability, and propagation under baseline and mechanical stress. RESULTS: Tissues lacking a defined pacemaker exhibited multiple initiation sites, irregular activation, and increased beat to beat variability, which worsened under mechanical stress. In contrast, HCN4 expressing pacemaker spheroids established a single stable initiation site, suppressed competing activity, and drove organized propagation in both NRVMS and human cardiomyocyte tissues. An optimal balance of depolarizing and stabilizing currents produced the most regular rhythm, while excessive alteration of inward rectifier potassium current impaired pacemaker control. These effects were preserved across tissue scales and were associated with increased markers of cardiac maturation during chronic pacing. CONCLSIONS: Cardiac pacemaker function is governed by ion channel balance and tissue architecture rather than channel expression alone. This in vitro platform provides a framework for studying rhythm instability and for developing biologically informed pacemaker and arrhythmia models relevant to pediatric and adult heart disease.

Public Health, Equity & Care Delivery

Evaluating Disparities in Pediatric Cochlear Implantation through Social Deprivation Index

Mahendran, Geethanjeli; Wright, Emily; and Govil, Nandini

Presenting Author: Emily Wright, BS, MD (Medical Resident)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 98

Abstract

Background: Cochlear implants (CI) provide children with profound sensorineural hearing loss access to sound, fostering speech and language development. This study investigates the role of the Social Deprivation Index (SDI), a composite measure of social deprivation, with higher scores associated with communities at risk for accessing healthcare. We hypothesized that children from communities with higher SDI scores would experience delays compared to children from low SDI communities, and that the COVID-19 pandemic would exacerbate these disparities. Methods: We conducted a retrospective review of pediatric patients (0–18 years) who underwent implantation at a tertiary children's hospital in a metropolitan area from December 2012 to September 2024. Demographic, clinical, and referral information was collected. SDI was assigned based on patient zip code and stratified as low (≤58) or high (>58). Pre- and post-pandemic (2020) groups were compared. Statistical analyses included chi-square and independent-samples t-tests, with significance set at $p < 0.05$. Results: Of 280 patients implanted, 222 met inclusion criteria. Mean age at diagnosis was 2.56 ± 2.62 years, and mean age at implantation was 4.01 ± 3.46 years. The cohort was 54.1% (n=120) female, 56.8% (n=126) White, and 33.3% (n=74) Black; 68.5% were publicly insured. Overall, 53.4% (n=119) were low SDI and 46.4% (n=103) high SDI. Across SDI groups, no significant differences were observed in age at diagnosis or implantation. However, post-pandemic high SDI patients were significantly older at diagnosis compared to pre-pandemic peers (3.16 ± 2.83 vs 2.03 ± 2.30 years, $p = 0.031$). Among Black patients living in vulnerable communities (high SDI), post-pandemic cohorts were significantly older at diagnosis (3.66 vs 1.21 years, $p < 0.001$), referral (4.95 vs 2.25 years, $p = 0.005$), and implantation (5.33 vs 2.49 years, $p = 0.003$), a disparity not observed in high SDI White patients. Conclusions: SDI highlights disparities in pediatric CI, particularly among Black children from high deprivation communities following the COVID-19 pandemic. These findings underscore the need for targeted interventions to improve timely diagnosis and implantation in socially vulnerable populations.

Neurodevelopment & Autism Interventions

EEG Foundation Modeling for Early Prediction of Infant Motor Development

Yang, David; and Xiao, Ran

Presenting Author: Ran Xiao, PhD (Junior Faculty)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 93

Abstract

BACKGROUND The Bayley Scales of Infant and Toddler Development (Bayley) are the gold standard for assessing early neurodevelopment. However, Bayley assessments are time-intensive, require specialized training, and are challenging to administer frequently, particularly in resource-limited settings. Although electroencephalography (EEG) provides an objective and biologically grounded window into brain development, infant EEG datasets remain scarce due to logistical and technical barriers to data acquisition. In contrast, adult EEG datasets are orders of magnitude larger. Recent EEG foundation models pretrained on adult data therefore present a unique opportunity to transfer learned neurophysiological representations to pediatric populations. OBJECTIVE To evaluate whether an adult pretrained EEG foundation model (CBraMod) can be adapted to predict infant composite motor scores, enabling scalable and objective developmental monitoring despite limited infant EEG data. METHODS We adapted CBraMod to infant resting-state EEG and evaluated progressive training strategies, including: (1) direct transfer of the adult-pretrained model (i.e., linear probing), (2) fine-tuning using downstream infant motor assessment data. The dataset consisted of longitudinal EEG recordings from 14 preterm infants across 37 visits (0–4 months adjusted age). All models were trained to predict composite motor scores derived from Bayley assessments, dichotomized into at risk for motor delay (<85, 1 SD below the age-matched mean) and within the expected range (≥85). Performance was evaluated using area under the receiver operating characteristic curve (AUROC) and compared against established EEG spectral biomarkers implemented with machine learning classifiers. RESULTS CBraMod achieved an AUROC of 0.75 under direct transfer, which improved to 0.79 with fine-tuning. Both approaches substantially outperformed the best baseline classifier trained on spectral power features (AUROC = 0.57). These findings demonstrate that pretrained adult EEG representations transfer effectively to infant EEG data. CONCLUSIONS Adult EEG foundation models can be successfully repurposed to predict infant motor development despite limited pediatric EEG availability. This EEG-based framework offers an objective and scalable complement to time-intensive behavioral assessments, enabling more frequent longitudinal monitoring and facilitating earlier detection of developmental risk, particularly in resource-constrained settings.

Infectious Disease & Immune Dysregulation

Ptpn11 mutations dysregulate trained immunity response in bone marrow-derived macrophages

Yoo, Erin; Yu, Wen-Mei; and Qu, Cheng-Kui

Presenting Author: Erin Yoo (Undergraduate student)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 77

Abstract

Background: Noonan syndrome (NS) is a relatively common genetic developmental disorder often diagnosed in children and is frequently caused by mutations in the Ras signaling pathway, nearly 50% of which are gain-of-function mutations in Ptpn11, a gene that encodes for SHP-2. Ptpn11E76K/+ (constitutively active SHP-2) mutant stem cells have also been associated with increased innate immune cell populations and elevated pro-inflammatory protein production due to aberrant activation of the innate immune system. Trained immunity is a functional state in which innate immune cells gain immunological memory following an initial stimulus. In this study, we hypothesized that the E76K mutant cells would exhibit dysregulated or overactivated trained immunity response.

Methods: The effects of Ptpn11 mutations on trained immune response was explored via a unique mouse model using Ptpn11E76K/+/Mx1-Cre+ (mutant) and Ptpn11+/+/Mx1-Cre+ (wild-type) mice. This mouse model was used to model NS to explore the cellular immune environment of NS patients. An in-vitro trained immunity model was performed on bone marrow-derived macrophages (BMDMs) using **β**-glucan as the primary training stimulus to establish trained immunity, followed by lipopolysaccharide (LPS) restimulation. Cytokine quantification, metabolic stress assays, and T cell proliferation assays were performed after trained immunity was established.

Results: All results displayed increased dysregulation of trained immunity in restimulated Ptpn11E76K/+ mutant cells compared to the controls. Trained mutant cells exhibited weakened immune and metabolic responses following restimulation, along with an overproduction of pro-inflammatory cytokines (i.e. TNF-**α** and IL-6), suggesting impaired immune function in the mutant cells.

Conclusions: Preliminary findings display a possible connection between gain-of-function mutations in Ptpn11 and trained immunity response, particularly following restimulation. Ptpn11E76K/+ mutant cells demonstrated reduced T cell proliferation, impaired metabolic function, and increased pro-inflammatory cytokine production, suggesting that NS patients may have dysregulated trained immunity response and basal immune function overall. Future studies may further clarify the molecular mechanisms and signaling pathways underlying trained immunity dysregulation in Ptpn11E76K/+ mutant cells.

Sickle Cell Disease & Transfusion Science

Optimization of stem cell fitness and mobilization using moderate transfusion and an oral anti-sickling agent in the sickle mouse model

Yoo, Justin; Wade, Jonathan; Patel, Ashwin; Goldsborough, Kennedy; Hernandez, Britney; Aliche, Mary; Wilson, Larry; Liotta, Dennis; Bunting, Kevin; Sheehan, Vivien

Presenting Author: Justin Yoo, MD, MS (Junior Faculty)

Rapid-Fire Information: Group 2 (1:45 pm - 2:15 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 105

Abstract

Ex vivo gene therapy (GT) in sickle cell disease (SCD) relies on mobilization of sufficient quantity and quality hematopoietic stem cells (HSCs) which are compromised by stress hematopoiesis, inflammation and disordered bone marrow (BM) stroma/vasculature. Currently, transfusions to a hemoglobin S% (%HbS) <30% is required to optimize HSC collection exposing significant number of blood units in a population at high risk for alloimmunization. We assessed if a moderate chronic transfusion therapy (CTT) regimen (%HbS <50%) or oral anti-sickling agent osivelotor modified HSC numbers, fitness, and mobilization. HbSS mice were treated with 4-5 weeks of 0.4% osivelotor chow or 8 weeks of CTT. Compared to untreated mice, osivelotor-treated mice had higher Hb (16 vs 8.3g/dL, p<0.001) without differences in BM cellularity. By flow cytometry, Lin-Sca-1+c-Kit+ (LSKs) and multipotent progenitor (MPP)-3 numbers trended lower in osivelotor-treated mice while MPP4 (p=0.04), short-term (p=0.03), and long-term HSCs (LT-HSCs) (p=0.03) were significantly lower compared to untreated controls consistent with reduced stress hematopoiesis. In the CTT arm, median Hb was 9.9g/dL and %HbS 41.2%. BM cellularity, LSKs and MPP subpopulations were similar to controls. We then compared HSC fitness with competitive transplants. BM cells from HbSS mice (CD45.2) on CTT, osivelotor, or no treatment were mixed with non-sickle (CD45.1) competitor cells and transplanted into irradiated recipients. Engraftment rates 25 weeks post-transplant were similar between osivelotor-treated and untreated sickle BM transplants while engraftment rates were higher with CTT than controls (p=0.01). When considering the number of LT-HSCs transplanted, osivelotor-treated and CTT-treated HSCs were 1.33-fold and 1.75-fold more efficient in engraftment than unmodified HSCs, respectively. Lastly, we assessed the impact of osivelotor on HSC mobilization with plerixafor. White blood cells and LSKs from peripheral blood trended higher in plerixafor-mobilized mice and significantly higher when conditioned with 8 weeks of osivelotor (p=0.01) compared to untreated controls. Osivelotor improved anemia, stress hematopoiesis, HSC fitness and mobilization with plerixafor. Although moderate CTT did not change HSC numbers compared to controls, likely due to persistent anemia, CTT-modified HSCs had higher engraftment efficiency. Less aggressive transfusions and an oral non-anti-sickling agent are potential alternatives to traditional CTT to optimize GT for SCD.

Cardiac Bioengineering & Platforms

Identifying Cardioprotective Compounds Against Carfilzomib-Induced Cardiotoxicity Using 2D High-Throughput Screening and 3D Human Cardiac Spheroids

Zhang, Wenhao; Patel, Gayatri; Wang, Matthew; Li, Stephanie; Vij, Sia;Du, Yuhong; Mandawat, Anant; Miller, Eric; and Xu, Chunhui*

Presenting Author: Wenhao Zhang, PhD (Postdoctoral Fellow)

Rapid-Fire Information: Group 1 (9:45 am - 10:15 am), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 61

Abstract

Background:Chemotherapy-induced cardiotoxicity remains a major clinical challenge, particularly for pediatric and young cancer survivors who face increased long-term cardiovascular risk. Carfilzomib (CFZ), a second-generation irreversible proteasome inhibitor used to treat relapsed or refractory multiple myeloma, is associated with a high incidence of cardiovascular adverse events. Despite this risk, effective cardioprotective therapies are limited, and there are currently no FDA-approved drugs to prevent chemotherapy-induced cardiac injury in pediatric patients, highlighting a critical unmet need. Methods:CFZ-induced cardiotoxicity was evaluated using human induced pluripotent stem cell–derived cardiomyocytes (hiPSC-CMs) in 2D culture. Cells were exposed to CFZ (0.6–10 μM, two-fold serial dilutions) for 48 hours. Tolerance to DMSO (vehicle) and to a 30-minute room-temperature incubation in cell suspension—simulating inter-lab cell transport—was also assessed. A streamlined high-throughput screening (HTS) protocol was developed to reduce processing time and compared with a traditional HTS method. Cell viability in 2D cultures was measured using CellTiter-Blue fluorescence. In parallel, assay adaptation to a 3D hiPSC-CM cardiac spheroid model was initiated, with viability assessed using a CellTiter-Glo luminescent assay. Results:CFZ induced significant, dose-dependent cytotoxicity in 2D hiPSC-CMs, with marked loss of viability at 5 and 10 μM after 48 hours; therefore, 5 μM CFZ was selected for downstream screening. hiPSC-CMs tolerated up to 1% DMSO without loss of viability, and a 30-minute room-temperature incubation did not affect cell survival, enabling practical cell transport. The streamlined HTS protocol reliably captured CFZ cardiotoxicity and demonstrated comparable performance to the traditional approach. Based on these findings, an HTS of approximately 5,000 bioactive compounds is underway using 5 μM CFZ as a lethal challenge and adrenalone as a cardioprotective positive control. In 3D cardiac spheroids, CFZ exposure (1 or 5 μM, 48 hours) resulted in >90% cell death, confirming severe cardiotoxicity in a tissue-like model. Conclusions:We established a robust and scalable platform to identify cardioprotective compounds against CFZ-induced cardiac injury. Incorporation of 3D cardiac spheroids enhances physiological relevance and translational potential, supporting accelerated discovery of cardioprotective therapies for chemotherapy-associated cardiotoxicity, particularly in pediatric populations.

Imaging & Diagnostic Tools

Orientation-Aware Diffusion Super-Resolution for 3T-Like Fetal MRI from Routine 1.5T Scans

Zhong Xinliu, Liu Ruiying, Lin Guohao, Huang Chuan, Goldman-Yassen Adam Ezra, Mehollin-Ray Amy Robben and Wang Yun

Presenting Author: Xinliu Zhong, BEng, MEng. (PhD Student)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 70

Abstract

BACKGROUND: Routine fetal brain assessment relies heavily on 1.5T MRI scanners due to their motion tolerance, but this comes at the cost of lower signal-to-noise ratio (SNR) and partial-volume blurring compared to 3T imaging. This "quality gap" obscures fine anatomical details and hinders downstream morphometric analysis. Existing super-resolution methods often fail to account for the complex, view-dependent artifacts inherent to anisotropic fetal HASTE acquisitions, leading to suboptimal smoothing or structural hallucinations.OBJECTIVE: To develop and validate an orientation-aware diffusion super-resolution framework that synthesizes diagnostic-quality 3T fetal brain contrast from routine 1.5T scans, bridging the resolution gap without sacrificing motion robustness.DESIGN/METHODS: We proposed a residual-shift diffusion framework combined with a Swin-UNet backbone (Fig. 1). A novel gated FiLM orientation encoder was introduced to explicitly condition the network on slice geometry (axial, coronal, sagittal), allowing it to adaptively invert view-specific anisotropies. Due to the lack of paired clinical data, training leveraged the FaBiAN numerical phantom to generate controllable high/low-resolution pairs. We employed a multi-level augmentation suite, including monotonic intensity remapping and "blackout" signal voids, to ensure generalization from synthetic training to clinical scans. The method was evaluated on both synthetic data and a clinical 3T dataset (simulated low-field) against state-of-the-art baselines like SwinIR and GAMBAS.Figure 1: Overview of our framework.RESULTS: On the synthetic test set, the proposed model achieved the best perceptual metrics (SSIM 0.81, LPIPS 0.042) compared to baselines. Downstream validation using Fetal-SynthSeg demonstrated consistent improvements in tissue segmentation accuracy, with Gray Matter Dice scores increasing by 28% relative to the input. On clinical data, the method achieved a Tissue Contrast T-score (TCT) of 0.94 (vs. 0.78 for input), indicating superior gray-white matter separability comparable to high-field imaging. Qualitative assessment confirmed the recovery of sharp gyral patterns and robustness against severe motion artifacts where competing models degraded.CONCLUSIONS: We present an orientation-aware diffusion framework that effectively bridges the domain gap between routine 1.5T acquisitions and diagnostic-quality 3T imaging. By explicitly modeling acquisition anisotropy and leveraging physics-informed synthetic training, this approach yields robust 3D reconstructions and precise tissue segmentation, offering a scalable solution for enhancing fetal neuroimaging reliability.

Cardiac Bioengineering & Platforms

Circulating Extracellular Vesicle miRNA Signatures as Noninvasive Biomarkers to Predict Fontan-Associated Liver Disease in Ovine Models

Zhou, Ashley; Takaesu, Felipe; Kievert, Jennifer; DeShetler, Cameron; Kelly, John; and Davis, Michael E.

Presenting Author: Ashley Zhou, BS

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 62

Abstract

BACKGROUND: Liver fibrosis is a common but poorly understood complication of Fontan-associated liver disease (FALD) that occurs post-Fontan procedure. Although elevated central venous pressure and chronic hypoxia are recognized contributors to liver stiffness, the molecular mechanisms driving FALD remain largely unknown. This knowledge gap limits the development of targeted therapies and leaves clinical solutions largely reactive. Circulating extracellular vesicles (EVs) in human serum carry RNA cargo, efficiently serving as noninvasive biomarkers with predictive power for FALD. In this study, we combine circulating EV-derived transcriptomics with machine learning approaches to identify RNA signatures associated with FALD, providing a novel method for early detection and risk stratification. METHODS: We employed a retrospective study design using clinically relevant ovine models of Fontan physiology previously established by our group. Serum samples were collected pre- and post-operatively in a matched-pairs design and paired with Doppler elastography to assess liver stiffness. Serum RNA profiles were sequenced by miRNA-seq and subjected to machine learning–based feature selection methods LASSO and Elastic Net. Differential gene expression analysis was utilized to identify conserved molecular miRNA markers predictive of FALD. RESULTS: Differential expression analysis identified four miRNAs that were significantly altered in the pre-operative condition, specifically hsa-miR-532-5p showing marked downregulation following the Fontan procedure. Both LASSO and Elastic Net feature selection models converged on the same set of 15 miRNAs, including hsa-miR-532-5p, that were strong predictors of liver stiffness, as assessed by liver elastography. Subsequent pathway enrichment analysis demonstrated that these miRNAs are involved in key inflammatory pathways, including activation of TGF-**β** signaling and the TAK1 complex. CONCLUSIONS: This study demonstrates that circulating EV–derived serum miRNA biomarkers are conserved across clinically relevant ovine Fontan models and strongly predict liver fibrosis. These findings provide the first translational evidence supporting EVs as a noninvasive biomarker source for early detection of FALD. Notably, hsa-miR-532-5p emerged as a potential repressor of hepatic stiffness and its downregulation post-Fontan could be contributing to the observed liver rigidity complication. By enabling identification of molecular predictors prior to fibrosis, this work establishes a framework for early detection and targeted therapies for FALD.

Infectious Disease & Immune Dysregulation

NGS Guided Pan Coronavirus Primer Set and top-down strategy for viral diagnostic toolkit development

Zhou, Yiyang (Tommy); Menachery, Vineet

Presenting Author: Yiyang (Tommy) Zhou, PhD (Research Scientist)

Rapid-Fire Information: Group 3 (4:00 pm - 4:30 pm), A148-A152-A156
Poster Information: Afternoon (4:45 pm - 5:45 pm), Room TBD
Poster Number: 78

Abstract

BackgroundThe continuous evolution of viruses challenges existing diagnostic and surveillance systems. Although next-generation sequencing (NGS) is the gold standard for tracking viral evolution, its complexity, cost, and infrastructure requirements limit its scalability for rapid public health response. Moreover, the strain- or variant-specific primer design offsets the need for affordability, speed, and accessibility during disease outbreaks. To address these limitations, we leverage upstream NGS approaches to create simplified, broad-spectrum molecular toolkits that target multiple viruses within the same family and remain effective as they evolve. Using coronaviruses as proof-of-principle, we refined a pan-coronavirus primer pool consisting of 28 "universal" primers. This platform offers rapid deployment to facilitate long-read sequencing, PCR based diagnostics, fluorescence-based detection, and targeted RNA enrichment in the context of an outbreak.Methods"Tiled-ClickSeq" is a click-chemistry based NGS platform that uses >300 SARS-CoV-2 single primers to sequence viral RNA. Building from this platform, we assessed cross-priming compatibility of SARS-CoV-2 primers with other coronaviruses and identified 28 pan-coronavirus primers to develop a viral family-based sequencing platform.ResultsUtilizing the >300-primer SARS-CoV-2 panel, we cross-sequenced coronaviruses HCoV-OC43, HCoV-229E, MERS-CoV, and SHC014-CoV. Bioinformatic analysis identifies 28 primers that consistently target conserved genomic regions across CoVs, enabling broad detection and characterization. We subsequently demonstrated that this primer pool could leverage long-read Nanopore sequencing to achieve complete or near-complete genome coverage of multiple coronaviruses. Building on this pan-Coronaviridae potential, we are developing detection and diagnostic strategies that prioritize cost-effectiveness and rapid deployment. These include: PCR- or LAMP-based pan-coronavirus diagnostics kits; fluorescent probe-based detection tools and coronavirus-specific RNA enrichment platform for basic and translational research. Conclusions / SignificanceThis proof-of-principle establishes a top-down strategy in which high-throughput NGS discoveries guide the development of simplified, and broad-spectrum, molecular tools for the detection of multiple coronaviruses. By using a selective pool of pan-coronavirus primers, this approach eliminates the need for strain- or variant-specific redesign. Such pan-viral primer systems enable rapid deployment and support early detection of emerging lineages. Although this initial work focuses on coronaviruses, the same NGS-guided framework is readily adaptable to other viral families and a pan-flavivirus panel is already in development.

Cardiac Bioengineering & Platforms

SLC25A1 Loss Alters the Cardiac Proteome During Postnatal Metabolic Maturation

Arefeayne, Nahum; Ohanele, Chiemela; Ghazal, Nasab; and Kwong, Jennifer

Presenting Author: Nahum Arefeayne, BS (MD/PhD Student)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 1

Abstract

BackgroundPostnatal cardiomyocytes undergo metabolic maturation, shifting toward fatty acid metabolism to sustain lifelong contractile demand. Prior work from our lab shows that systemic loss of Slc25a1 in mice disrupts embryonic cardiac metabolic maturation, implicating SLC25A1 as a regulator of mitochondrial-centered developmental programming. Whether SLC25A1 also governs maturation after birth, a window defined by mitochondrial expansion and proteome remodeling, remains unknown. We hypothesize cardiomyocyte SLC25A1 is required for postnatal metabolic maturation and cardiomyocyte-specific deletion (Slc25a1-cKO) will prevent metabolic maturation.MethodsWhole hearts from cardiomyocyte-specific SLC25A1 deletion mice and controls were collected at P28 (n=5/genotype) and analyzed by TMT-based LC-MS/MS proteomics. Secondary analyses included pathway enrichment (GO/KEGG/Reactome; ORA and GSEA) and targeted evaluation of mitochondrial oxidative metabolism and lipid handling.ResultsAcross P28 hearts, quantitative proteomics identified 71 differentially abundant proteins using preset thresholds of (q£0.1, llog2FCl3 0.6), with a strong bias toward decreased abundance (59 downregulated vs. 12 upregulated in Cre vs. WT). Downregulated proteins consisted of mitochondrial metabolic machinery, including ETC/OXPHOS subunits and ATP synthase components. OXPHOS-complex annotated heatmaps showed a decrease across multiple respiratory chain complexes in Slc25a1-cKO hearts. Enrichment analysis supported this pattern: GO Biological Process over representation analysis highlighted terms related to cellular respiration, oxidative phosphorylation, and ATP synthesis coupled electron transport while Reactome/GSEA demonstrated negative enrichment of aerobic respiration and electron transport chain. Similarly, Hallmark analysis of OXPHOS pathways displayed global negative enrichment, indicating broad downregulation of the oxidative phosphorylation program in Slc25a1-cKO hearts. Leading edge analysis of lipid GO terms revealed an increase in proteins involved in fatty-acid b-oxidation, acyl-CoA metabolic processes, lipid localization and lipoprotein remodeling.ConclusionsAt P28, when metabolic maturation in cardiomyocytes should be complete, SLC25A1 loss produces widespread proteome remodeling in the mouse heart. This is characterized by coordinated suppression of mitochondrial oxidative phosphorylation/respirometry machinery alongside induction of lipid-handling and stress response programs. These data support a model in which SLC25A1 is required to establish or maintain the mature metabolism proteome after birth. Together the proteomic signatures indicate impaired OXPHOS maturation and altered lipid homeostasis as key downstream axes linking citrate transport to postnatal cardiac metabolic vulnerability.

Oncology & Cellular Therapy

Symptom Profiles and Associated Biopsychosocial Risk Factors during Pediatric Cellular Therapy: A Secondary Analysis

Basha,Mays; Ward, Jessica; Kaplan, David; Kwekkeboom, Kristine; and Montgomery

Presenting Author: Mays Basha, PhD (Postdoctoral Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 23

Abstract

Background: Advances in cellular therapy, including hematopoietic stem cell transplantation (HSCT) and chimeric antigen receptor T-cell (CAR-T) therapy, offer potentially curative treatment for the children living with life-threatening malignant and non-malignant conditions. Pediatric patients undergoing cellular therapy experience varying physical and psychological symptoms. Identifying subgroups of patients who experience multiple symptoms can inform targeted interventions. However, traditional analytic approaches often assume homogeneity within patient populations, overlooking distinct subgroups that share similar symptom experiences.Purpose:This study aimed to identify symptom burden profiles among pediatric patients undergoing HSCT or CAR-T therapy, along with demographic and clinical factors linked to profiles.Method: We conducted a secondary analysis of previously collected data from a multisite study of symptoms in children undergoing HSCT and CAR-T therapy (N = 131). Latent class analysis (LCA) was used to identify symptom profiles and logistic regression evaluated demographic and clinical predictors of these symptom profiles.Result: LCA identified two distinct symptom profiles (high symptom burden and low symptom burden) across all 30 symptoms. Further, two profiles were identified within symptom subscales (high and low physical symptom burden and high and low psychological symptom burden profiles). Approximately half (53.06%) of patients belonged to the high symptom burden profile using all 30 symptoms. Within subscales, a majority of patients were classified into the high physical symptom burden profile (63.1%) and the low psychological symptom burden profile (61.36%). Undergoing HSCT ($p = 0.04$) was associated with an increased risk of being in the high physical symptom burden profile. In contrast, older age ($p = 0.014$) was significantly linked to an increased risk of being in the high psychological symptom burden profile. None of the other demographic or clinical variables were associated with the symptom burden profiles.Discussion: This study is among the first to use LCA to identify symptom burden profiles and examine related demographic and clinical factors. Distinct symptom patterns emerged during cellular therapy. Only HSCT and older age influenced high symptom burden profiles. Future research should explore symptom-specific interventions, like physical management for HSCT recipients and psychological support for older adults.

Public Health, Equity & Care Delivery

Increasing Utilization of the General Movements Assessment in the NICU for Early Identification of Abnormal Movement Patterns: A Quality Improvement Initiative

Botbyl, Rachel; Barnes, Kaleb; Maitre, Nathalie; and Neel, Mary Lauren

Presenting Author: Rachel Botbyl, MD, MSCR (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 35

Abstract

Background: Early identification of infants at risk for neurodevelopmental delays remains a challenge. The General Movements Assessment (GMA) is a validated tool for detecting early neurological impairment, particularly cerebral palsy (CP). Cramped synchronized or absent fidgety movement patterns are rare but require systematic performance and response to ensure appropriate medical care for these children. Despite GMA utility, barriers including logistical complexity, inconsistent communication, and unclear referral processes limit its use in the NICU. Methods: We aimed to increase GMA performance from 32% to 64% among eligible infants and to increase appropriate referrals for infants with abnormal GMAs to 98% from August 2023-February 2025 in our large level IV NICU. We used IHI methodology with an initial barriers and facilitators survey to build a key driver diagram to guide two 6-month Plan-Do-Study-Act cycles, based on interventions derived from a prioritization matrix exercise. Baseline data were collected monthly for 6 months. Cycle 1 focused on GMA faculty/staff education and culture. Cycle 2 addressed operational challenges and provider comfort with delivering unfamiliar diagnostic reports.Results: During baseline, n=56 infants were eligible, and 32% of those received a GMA. 83% of those with abnormal GMA were appropriately referred for MRI and Developmental Progress Clinic. During Cycle 1, n=53 infants were eligible, 48% received a GMA, and 89% were appropriately referred. During Cycle 2, n=60 infants were eligible, 64% received a GMA, and 100% were appropriately referred. Conclusion: Following 2 PDSA cycles, we have successfully increased GMA completion among eligible NICU infants and maintained high referral rates for those with concerning findings. Interventions focused on communication and provider education proved effective. Sustaining improvements and achieving complete screening of eligible patients will require continued education, multidisciplinary collaboration, and integration into routine NICU processes. Preparation for a third intervention cycle is ongoing, with plans to address respiratory support needs as a possible barrier to GMA completion.

Oncology & Cellular Therapy

Single Institution Analysis of Structural Variants of Multifocal Wilms Tumor

Burstein, Shana; Sutton, Kathryn; Tsuchiya, Karen; Akkari, Yassmine; Yin, Hong; Hong, Andrew

Presenting Author: Shana Burstein, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 24

Abstract

Background: Wilms tumor (WT), the most common pediatric renal tumor, is generally associated with excellent outcomes with 90% overall survival. A minority of cases (5-10%), including the presence of bilateral (BWT) or multifocal WT, are associated with worse prognoses. These tumors are thought to arise from genetic predisposition and are more commonly associated with pediatric syndromes including Beckwith-Wiedemann, WAGR and WT1 syndromes.There are multiple well-studied molecular mechanisms leading to the development of BWT/multifocal WT, as well as several rarer genomic variants that have been discovered as access to robust sequencing improves. At least 29 genes or epigenetic changes have been identified in association with BWT/multifocal WT, but over 50% of patients with BWT or multifocal WT lack a detectable genetic cause. There is significant genetic heterogeneity across WTs, which we hypothesize is also reflected across tumor foci in multifocal WT. Methods: An IRB-approved retrospective chart review was performed to identify all patients with multifocal WT at Children's Healthcare of Atlanta (CHOA) from 2008-2025 using EPIC SlicerDicer. Tumor and adjacent unaffected kidney tissue were isolated from surgical specimens obtained for prior clinical care. All eligible samples will undergo targeted microarray analysis/genomic studies to assess for structural variants within the multifocal tumor tissue. We will assess for known risk factors including chromosome (chr) 11p15 loss of imprinting/loss of heterozygosity (LOH), LOH at chr 1p, 16q and 17p and gain of chr 1q. Results: 30 patients with multifocal WT were identified at CHOA. After completing the microarray/genomic studies, we will report the spectrum and frequency of known and novel copy number variants present within multifocal WT samples. Conclusions: There remains a gap in the understanding of the genetics of multifocal WT given its rarity. We reported a substantial single-institution cohort of patients with multifocal WT, and anticipate that sequencing these tumor specimens will offer insight into the pathogenesis of multifocal WT. By expanding our knowledge of WT genetics, we can more effectively stratify patients by tumor genetics, eliciting which patients may require treatment escalation or de-escalation, and may also identify novel, targeted treatment options for high-risk WT.

Neurodevelopment & Autism Interventions

Exploring VB-MAPP Skill Profiles as Predictors of Speech Generating Device Proficiency in Autistic Children

Callahan, Caitlin; and OGuinn, Nicole

Presenting Author: Caitlin Callahan, BS, MS

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 16

Abstract

Approximately 25–30% of individuals with autism spectrum disorder (ASD) are considered minimally verbal or nonverbal, relying on alternative communication methods such as picture exchange systems or speech-generating devices (SGDs; Golden Care Therapy, 2024). Understanding the skills that support successful SGD learning can help clinicians individualize interventions and improve communication outcomes. This study examines whether pre-intervention VB-MAPP skill profiles are associated with the time to acquire a SGD, aiming to identify foundational skills that contribute to faster device learning. A chart review was conducted of Marcus Autism Center patients (n = 46). The study inclusion criteria required that the participant completed a protocol aimed at increasing independent requesting using an SGD and reached mastery criteria (80% independent requesting across 3 consecutive sessions) and had a VB-MAPP assessment completed within 6 months prior to beginning intervention to acquire an SGD. A full multiple linear regression model including all VB-MAPP subdomains was initially tested. To address potential multicollinearity and improve interpretability, a reduced model including only the strongest predictors (i.e., Tact, Linguistics, LRFFC, Echoic, and Group) were then evaluated. Linear regression did not identify significant independent predictors of latency to master using an SGD, likely due to multicollinearity among VB-MAPP subdomains. Correlation analyses suggest that higher pre-SGD language and academic skills trend toward faster mastery, whereas baseline requesting scores are weakly associated with longer time to mastery. A Principal Component Analysis revealed two primary dimensions of the VB-MAPP subdomains: one reflecting social-communication skills (e.g., Play, Social, Imitation) and another representing cognitive-academic abilities (e.g., Reading, Math, Linguistics). Future research will aim to broaden the scope of this study by including individuals who have learned alternative communication modalities, such as picture exchange communication systems (PECS) and pointing as a communication modality. By identifying the domains most strongly associated with successful SGD acquisition, practitioners can prioritize prerequisite skill development and select the most suitable communication modality for each learner. Ultimately, this work contributes to advancing evidence-based, data-informed decision-making in augmentative and alternative communication (AAC) training.

Neurodevelopment & Autism Interventions

Aberrant microRNA Regulation and Cellular Stress in iPSC-Derived Microglia From 22q11.2 Deletion Syndrome

Cheng, Leah; Michalski, Christina; Parker, David; Niu, Weibo; Wang, Jianjun; Imes, Sidney; Cubells, Joe; Duncan, Erica; and Wen, Zhexing

Presenting Author: Leah Cheng, BS (Research Specialist)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16501 and 16502
Poster Number: 87

Abstract

22q11.2 deletion syndrome (22qDS) is a genetic disease that has been shown to be associated with a range of neurodevelopmental phenotypes, including intellectual disability, attention-deficit/hyperactivity disorder (ADHD), and autism spectrum disorder. Children with 22qDS have elevated risk for anxiety disorders and also for schizophrenia spectrum disorders as they step into young adulthood. Despite the clinically observed symptoms, the molecular mechanisms linking 22qDS to neurodevelopmental deficits remain incompletely understood. Around 50 genes are located in the 22q11.2 deletion region, including DGCR8, which is known to play a key role in microRNA (miRNA) biogenesis. miRNA are expressed in a cell-type specific manner and regulate gene expression post-transcriptionally. In microglia, brain-resident immune cells important for neuronal homeostasis, disruption of miRNA biogenesis leads to DNA damage and hyperactivation. We therefore hypothesized that 22qDS-caused haploinsufficiency of DGCR8 leads to reduced production of miRNA, further resulting in hyperactivation of microglia, promoting aberrant synapse pruning and disrupting neuronal development. Here, microglia were differentiated from induced pluripotent stem cells (iPSCs) derived from four individuals with 22qDS and three age- and sex-matched healthy controls. We performed both miRNA and mRNA sequencing to characterize transcriptional and post-transcriptional changes associated with the deletion. Our analyses revealed selective dysregulation of miRNA expression in 22qDS microglia, with subsets of miRNAs showing significant upregulation or downregulation, indicating that the 22q11.2 deletion exerts a non-uniform impact on miRNA biogenesis. Integration of miRNA and mRNA datasets using the MIMR tool revealed inverse regulatory relationships, with downregulated miRNAs corresponding to upregulated target mRNAs and upregulated miRNAs corresponding to downregulated targets in 22qDS microglia. Gene set enrichment analysis of expressed mRNAs demonstrated significantly reduced expression of genes involved in mitochondrial metabolic processes, while pathways related to cell cycle regulation, DNA repair, and cellular response to stress were upregulated in 22qDS microglia. Together, these findings suggest that as miRNA levels are altered in 22qDS microglia, cellular functions including stress response and DNA repair are meanwhile activated. These changes may contribute to microglial activation and eventually disrupt neurodevelopment and underlie neuropsychiatric risk.

Data Science, Methods & Biomarkers

Analysis of Difficult Airway Clinical Decision Support for Pediatric Inpatient Providers

Cowart, J Harrison; Popkin, Melissa; Thompson, Sarah; Kandaswamy, Swaminathan; Yarahuan, Julia KW

Presenting Author: Harrison Cowart, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 51

Abstract

IntroductionPatients with difficult airways (DA) have a significant risk of rapid decompensation. Our organization defines a DA as an intubation attempt requiring more than two attempts or a known anatomic abnormality. The protocol for patients with DAs includes clinical decision support (CDS) tools to aid provider awareness, perception, and process knowledge. We performed pre-intervention and post-intervention surveys to evaluate provider performance in these three domains and redesigned our CDS tools with a needs-informed perspective. MethodsProviders were prospectively surveyed regarding two actively admitted patients, one with and one without a DA label. Inclusion criteria involved providers of various training levels and pediatric non-surgical specialties taking care of inpatients. For both patients, a hypothetical clinical scenario with respiratory decompensation was proposed with the possible decision to call a difficult airway response team (DART) alert. This survey was performed prior to and following the implementation of standardized intubation input with discrete data elements ("Smartform") and an alert redesign. A multidisciplinary team met biweekly to redesign the clinical decision support based on the failure modes identified. DA designations were tracked over time. Statistical analysis performed with Chi-square and Student's t-test. ResultsThe pre-intervention group contained 22 participants, while the post-intervention group contained 10 participants. There was no statistically significant difference in the answers to questions regarding awareness and perception of the intervention. The post-intervention cohort did not incorrectly call a DART alert in any of the surveys compared to 18% of the pre-intervention cohort. The median number of DA labels added per month was similar between pre-intervention (n =21 months) and post-intervention (n = 7 months) time periods (10 vs 14, p=0.74); however, the median number of DA labels removed per month was significantly different (2 vs 5, p=0.01). ConclusionThe improvement in DA label removals, stable DA label additions, and the trend towards decreasing the number of incorrect DART activations is promising. These findings reflect a non-inferior DA alert with high-value care potential. Our findings may suggest an improvement in the process knowledge of providers who are interacting with the new alert and the awareness to reevaluate patients regarding their airway status.

Oncology & Cellular Therapy

Eculizumab as a Therapeutic Strategy for Severe Hyperhemolysis and Organ Dysfunction in Sickle Cell Disease

Dave, Ronak; Westbrook, Adrianna; Pickett, Logan; Graciaa, Sara; Briones, Michael; Yee, Marianne; Fasano, Ross; Schoettler, Michele L.; and Chonat, Satheesh

Presenting Author: Ronak Dave, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 25

Abstract

BackgroundHyperhemolysis (HH) is a life-threatening complication of sickle cell disease (SCD). Emerging evidence suggests that complement activation plays a key role in the pathogenesis of HH. Although limited case reports and series indicate that eculizumab, a C5 complement inhibitor, may be an effective therapeutic option, larger studies are needed to better characterize its efficacy and safety in a functionally asplenic patient population. This study describes the CHOA experience with complement inhibition for SCD HH. MethodsIn this IRB-approved single-institution retrospective study, we identified patients with SCD treated with eculizumab for HH (defined as rapid hemoglobin decline to below pre-transfusion value in delayed hemolytic transfusion reaction (DHTR) or a drop in hemoglobin ≥ 3.0 g/dL below baseline in non-DHTR) at CHOA from 8/1/17 to 3/19/25. Our institution uses a standardized guideline for eculizumab-based management of HH. ResultsForty episodes of HH treated with eculizumab across 28 individuals were identified. The median age at the first dose of eculizumab for each episode was 16 years (range 2-20 years). Most episodes occurred in HbSS genotype (n=38, 95%) and in females (n=25, 63%). The most common triggers for HH included DHTR (n=19, 48%), vaso-occlusive episodes (n=9, 23%), acute chest syndrome (n=9, 23%), and infection (n=2, 5%). The median drop in hemoglobin from baseline was 4.2 g/dL, and hemoglobin nadir ranged between 1.9-7.2 g/dL. Organ dysfunction occurred in 31 (78%) episodes, including hemodynamic instability requiring pressors (n=8, 20%), need for respiratory support (n=23, 58%), renal injury (n=13, 33%), or hepatic injury (n=22, 56%). Estimated 1-year OS from admission was 78% (95% CI 56-90%). The median number of eculizumab doses for HH was 1 (range 1-5). In the 6 months following the last eculizumab dose, no patients experienced invasive infection. ConclusionThis study represents the largest reported series of eculizumab use for HH in SCD and demonstrates its safety and tolerability profile in a population with a high burden of organ dysfunction. Given the frequency of severe, multi-organ dysfunction in HH and the limited therapeutic options available, these findings support the role of complement inhibition as an important treatment in critically ill patients with HH.

Data Science, Methods & Biomarkers

Assessing the Performance of Large Language Models for Diagnostic Reasoning for Primary Ciliary Dyskinesia in Pediatric Patients

Fain, Mary Ellen; Rajwal, Swati; Diaz-Asper, Kiri; Silva, George Lucas; Sarker, Abeed; and Guglani, Lokesh

Presenting Author: Kiri Diaz-Asper (Undergraduate Student) on behalf of Mary Ellen Fain, MD (Medical Fellow)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16501 and 16502
Poster Number: 83

Abstract

Background: Primary Ciliary Dyskinesia (PCD) is a rare genetic disorder with heterogeneous clinical presentation that lacks a diagnostic gold standard. Currently, diagnosis relies on clinician interpretation of multiple tests, as symptom overlap with other conditions complicates diagnosis based on clinical features alone. Given diagnostic complexities and increasing availability of large language models (LLMs), providers and families are more likely to seek guidance from LLMs. Therefore, it is essential to assess the accuracy and reliability of widely available LLMs in considering PCD evaluation. Methods: We evaluated four open-source LLMs, and an ensemble approach based on consensus between the 4 LLMs, on ability to accurately recommend PCD diagnostic testing. Each model analyzed 28 de-identified initial pulmonology clinic visit notes from patients later diagnosed with PCD. For each case, the model indicated whether further PCD evaluation was warranted (yes/no/uncertain) and provided brief rationale with suggested diagnostic tests. Because all cases had a confirmed PCD diagnosis, only sensitivity (proportion of correctly identified cases warranting further testing) was assessed. Results: Sensitivity between the 4 LLM models varied significantly ($p < 0.001$), and ranged from 0.48 - 1.00, with model Mistral-7B performing the best and GPT OSS 120B performing the worst. All models except GPT OSS 120B, as well as the ensemble majority vote, performed significantly better than chance ($p < 0.05$). Llama 3 70B, Qwen 2.5 7B, Mistral-7B, and the ensemble exceeded a sensitivity threshold of 0.80. Conclusions: In screening for PCD, high sensitivity is preferred as false negatives are more problematic than false positives. Although optimal sensitivity thresholds for LLMs are poorly defined, standard PCD screening tests typically achieve sensitivity of 0.80-0.98. Thus, our finding of LLMs with sensitivities exceeding 0.80 may support their use as effective screening tools. Interestingly, our results also suggest smaller models may offer comparable utility to larger ones in PCD screening, which has implications for resource-efficient clinical AI applications. Further studies with larger, more diverse datasets are needed to validate LLM-based identification of PCD. Nevertheless, LLMs show promise as supportive tools for clinically complex cases where diagnostic workup can be optimized based on available resources and further narrowing of differential diagnoses.

Gastrointestinal, Hepatic & Renal

Successful JAK Inhibition Treatment of Very Early Onset Inflammatory Bowel Disease Associated with A De Novo Monogenic Mutation in CUL3

Geiculescu, Irina; Diefendorf, Caitlin; Fitch, Taylor; and Rudra, Sharmistha

Presenting Author: Caitlin Diefendorf, BS, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 7

Abstract

Background: Very early onset inflammatory bowel disease (VEO-IBD) is a chronic condition of the gastrointestinal (GI) tract characterized by a dysregulated immune system diagnosed in patients under six years old. Inflammation arises from immunomodulatory monogenic and polygenic defects contributing to clinical heterogeneity and the need for alternative therapeutic approaches. Methods/Case Presentation: The male patient born premature at 30 weeks presented to GI clinic at 2-years-old for constipation and severe malnutrition, with history of ear infections, autism, and developmental delay. He had an elevated ESR, normal CRP, and an elevated fecal calprotectin of 291 ug/g. Endoscopy showed an edematous ileocecal valve. Pathology revealed intraepithelial eosinophils in the esophagus, increased crypt apoptosis in the descending colon, and lymphoplasmacytic inflammation in the lamina propria. Immune studies showed normal IL-18, elevated IL-6 and IL-8, decreased FOXP3 and DHR, low pneumococcal titers, and a de novo variant in Cullin 3 (CUL3) on exome sequencing. He was diagnosed with VEO-IBD and following initiation of ruxolitinib, he had improved stool consistency/frequency, resolved vomiting, and improvement in straining during bowel movements. He gained weight and a repeat fecal calprotectin improved to 182 ug/g. Results/Discussion: CUL3 mutations cause impaired ubiquitin proteasome degradation of the NF-κB complex, and thus stabilization and gene transcription of pro-inflammatory proteins that ultimately cause sustained activation of the JAK/STAT pathway. Ruxolitinib inhibits the JAK/STAT pathway leading to reduced expression of pro-inflammatory cytokines, such as IL-8. This mechanism is likely the cause for the resulting improvement seen in this case. Similar mechanisms have been reported with gene mutations such as NLRP12 and UBA1 (as seen in VEXAS syndrome). Ruxolitinib has previously been shown to improve clinical outcomes in two monogenic causes of IBD; a polymorphism in the PTPN2 locus and a gain of function mutation in STAT3. Conclusions: We report a novel variant of VEO-IBD which demonstrated significant clinical and biochemical improvement following treatment with the JAK inhibitor, ruxolitinib. Ruxolitinib was chosen because it inhibits the signaling pathway that is overamplified by the de novo CUL3 mutation. This case highlights the therapeutic potential of JAK inhibition in novel genetic mutations implicated in monogenic VEO-IBD.

Infectious Disease & Immune Dysregulation

Pediatric TB Disease Severity in the State of Georgia: A Fifteen Year Retrospective Review

Dubick, Collin; Kempker, Russell; and Schechter, Marcos

Presenting Author: Collin Dubick, MD (Medical Fellow)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16403 and 16404
Poster Number: 72

Abstract

BACKGROUNDStudies suggest that most children have nonsevere TB, but this has not been evaluated in the United States. The identification of nonsevere TB enables a shortened 4 vs 6-month rifampin, isoniazid, pyrazinamide, and ethambutol (RIPE)-based treatment regimen for children with drug-susceptible disease—a new standard of care established by the landmark 2022 SHINE trial. We hypothesized that most pediatric TB in the state of Georgia is nonsevere and conducted a statewide retrospective review of medical records of children with TB to inform the impact of implementation of shortened TB treatment.METHODSThis study is a retrospective review of the medical records of children ≥3 months and ≤15 years of age diagnosed with TB disease in Georgia from Jan 1 2008-Dec 31 2023. TB disease severity, the primary outcome, was determined by manual review of medical records and designated as nonsevere per the SHINE trial protocol, probable nonsevere if consistent with nonsevere TB despite absent microbiologic results, severe for all other TB disease forms, and indeterminate if not determined. Descriptive statistics were used to summarize key demographic and clinical variables associated with disease severity. Chi-squared testing or Fisher's exact test and Wilcoxon rank sum test were used as appropriate to analyze statistical differences according to disease severity. RESULTS357 medical records were included in this study. Based on assessment, 99 (28%) TB diagnoses were consistent with nonsevere disease, 76 (21%) with probable nonsevere disease, 130 (36%) with severe disease, and 52 (15%) with indeterminate disease severity. Excluding 52 diagnoses of indeterminate severity, 175 (57%) of diagnoses were consistent with either nonsevere or probable nonsevere disease.According to disease severity, there were significant differences in age group, TST results, symptoms, culture results, disease type, and treatment duration.Median treatment duration for nonsevere and probable nonsevere disease was 27 weeks. We estimate that implementing four-month RIPE treatment in Georgia would save approximately 11 weeks of therapy per child with nonsevere TB per year. CONCLUSIONMost children in Georgia appear to have nonsevere TB and qualify for a four-month RIPE-based regimen. Future studies should focus on implementation of this regimen.

Reproductive, Adolescent & Women's Health

Aprepitant versus Traditional Anti-Emetic and Adjunctive Therapies for Cannabinoid Hyperemesis Syndrome in Adolescents

Durham, Rebecca; Steck, Alaina; and Yarahuan, Julia

Presenting Author: Rebecca Durham, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 43

Abstract

Background: Cannabinoid hyperemesis syndrome (CHS) is a common cause of recurrent hospital admissions. Fosaprepitant (FA) and aprepitant (AP) are atypical antiemetics used at our facilities to treat refractory CHS, though little information is known regarding use of these medications for this. We aim to analyze the demographics among patients who received these medications compared to those who did not. We also aim to determine the differences in outcomes between these groups including length of hospital stay (LOS). Methods: Patient admissions to the hospital for CHS from July 1,2023 - June 30,2025 at all Children's Healthcare of Atlanta hospitals were identified using billing diagnoses within Epic, the electronic health record. Epic application Slicer Dicer was used to identify antiemetics administered during each encounter. Microsoft Excel was used to analyze demographics and outcomes among admissions and patients who did and did not receive FA or AP. Chi-squared tests were performed to compare categorical variables. Two-tailed two-sample t-tests were implemented for quantitative data.Results: There were 226 hospital admissions and 125 unique patients with CHS diagnoses identified. FA or AP was administered during 57% of encounters. Demographic data demonstrated admissions where FA or AP was administered were more likely among patients who were older (18.3 vs. 17.4, p=1.2E-07), female (81 vs 61%, p=8.3E-04), and white (60 vs 37%, p=8.7E-04). There were no significant differences when patients rather than admissions were compared. Patients who received FA or AP were more likely to receive more total (16 vs 10, p=7.8E-03) and unique (5 vs 4, p=3.0E-04) antiemetics during admission. The shortest time from first administration of an antiemetic to discharge was observed with FA or AP to discharge (70 hours). Conclusions: Demographic disparities among CHS admissions were noted between patients who did and did not receive FA or AP. Over half of admissions for CHS involved FA or AP administration. These admissions had a shorter time to discharge compared to other antiemetics analyzed in this study. Clinical leaders should consider revising policies that limit clinician access to these medications, as providing FA/AP earlier in hospitalization may shorten LOS and decrease costs, though further research is needed.

Cardiac Bioengineering & Platforms

Very High Donor-Derived Cell-Free DNA in Pediatric Heart Transplant Recipients

Haq, Khadijah; Elutilo-Ayoola, Temitope; Shi, Jing; Shen, Ling; Wassimi, Maira; Mao, Chad; Jandu, Simran; Mahle, William; and Butto, Arene

Presenting Author: Temitope Elutilo-Ayoola, BA, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 2

Abstract

Very High Donor-Derived Cell-Free DNA in Pediatric Heart Transplant RecipientsBackground: Elevated donor-derived cell-free DNA (dd-cfDNA) may reflect graft injury, but outcomes of pediatric heart transplant (HT) recipients with very high dd-cfDNA are unknown. We sought to characterize outcomes of this population at our center.Methods: We retrospectively studied children post-HT monitored with dd-cfDNA (AlloSure, CareDx, Inc.) from 6/2022 to 4/2025 with at least 3 months of follow-up. Analyses began when dd-cfDNA first exceeded 1%, a threshold reported to be associated with a substantial risk of mortality in a study of adult HT recipients. We used a composite endpoint of graft dysfunction (ejection fraction < 45% or > 10-point decline, restrictive physiology defined as end-diastolic pressure > 15 mmHg with cardiac index < 2.0 L/min/m², or new heart-failure hospitalization), acute rejection, or death. Cox proportional hazards modeling was used for analysis.Results: We examined 35 HT recipients (69% cardiomyopathy, 31% congenital, 29% ventricular assist device bridge). Of 417 samples obtained a mean of 5.6 ± 4.4 years post-HT, 32.9% measured ≥ 1%. Most were obtained during asymptomatic routine surveillance (91.1%). During follow-up, 31.4% developed graft dysfunction, 54.3% had rejection, and 22.9% died. There was no difference in demographics, underlying heart disease, crossmatch positive HT, or subtherapeutic tacrolimus levels between those who reached the composite endpoint (n=14) and those who did not (n=11). Patients who reached the endpoint were older at the time of HT (median 10.6 vs 2.7 years, HR 1.12, 95% CI 1.01–1.23, p=0.025) and most (79%) developed new or higher donor-specific antibodies (Table). Patients who reached the endpoint also had a higher percentage of samples with dd-cfDNA > 1% (67% vs 30%, p < 0.001, Figure). Conclusion: Very high dd-cfDNA may identify pediatric HT recipients at greater risk for future graft dysfunction or rejection. Further studies should define how the timing and persistence of these elevations influence graft health and long-term outcomes.

Neurodevelopment & Autism Interventions

Adapting a Caregiver Well-Being and Behavioral Intervention for Autistic Children in Western Kenya: Development of a mHealth Peer-Delivered Program

Fauntleroy-Love, Kristin; Baumberger, Geneva; Saina, Chelegat; Rispoli, Mandy; McHenry, Megan; and McNally Keehn, Rebecca

Presenting Author: Kristin Fauntleroy-Love, MBBS (Junior Faculty)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 17

Abstract

Background: In low- and middle-income countries (LMICs), limited access to autism knowledge and interventions contribute to stigma and poor outcomes. Caregivers need support to improve their autism knowledge and reduce stress and isolation. The objective of this study was qualitatively describe the adaptations made during co-development and early implementation of Takia, a mobile health (mHealth) autism education and peer support program for caregivers of autistic children in western Kenya.Methods: Takia was developed by adapting the content and format of a caregiver well-being and behavioral intervention program developed by our interdisciplinary US-Kenya research team. Weekly team meetings and two program development sessions with 10 Kenyan caregivers of autistic children were held to develop, adapt, and refine Takia content. Iterative adaptations to Takia throughout the co-development and early implementation process were coded using the Framework for Reporting Adaptations and Modifications-Expanded. The final program included nine structured educational modules delivered via WhatsApp covering basic autism education, caregiver coping strategies, and behavioral interventions. Ten caregivers were trained as Autism Information Navigators (AIN) and provided education and peer support to caregivers and community members. Implementation data were collected and qualitatively summarized via AIN logs and Google Analytics. Results: Thirty-two adaptations were made during development; 78% (n=25) were guided by caregivers. Key adaptations included expanding the curriculum text), reordering modules, and adding videos with educational content and caregivers stories. Additionally, specific Takia modules were identified as modules to deliver to general community members to increase autism awareness. In the initial 5 months, AINs have delivered Takia to 18 caregivers and provided autism education to 511 community members. To date, 99 active users have accessed the Takia modules online. Conclusions: Takia's co-development process highlights the importance of engaging caregivers in developing and implementing culturally grounded interventions. This work lays the foundation for further scale up and implementation of sustainable, community-driven interventions to fill critical autism resource gaps in LMICs.

Imaging & Diagnostic Tools

Assessing the Impact of MR Image Quality on Automated AI-Driven Pediatric Glioma Lesion Segmentation Performance

Flannery, Brennan; Valappil, Fareeda Farhan Naduvil; "and" Viswanath, Satish E.

Presenting Author: Brennan Flannery, PhD (Postdoctoral Fellow)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16403 and 16404
Poster Number: 67

Abstract

Background: Pediatric gliomas represent the most common childhood brain tumors, which are often identified and characterized using magnetic resonance imaging (MRI) to inform cancer staging and treatment decision making. However, accurate lesion delineation on routine MRI remains challenging due to heterogeneous appearance and anatomical differences from adults. Recent advances in AI-based deep learning models (DLMs) have demonstrated strong generalizability across diverse adult imaging cohorts, but their performance in pediatric neuro-oncology remains poorly understood. Transferring such models to the pediatrics could catalyze development of image-based markers toward precision oncology. This study systematically benchmarks two segmentation DLMs for pediatric glioma segmentation on MRI, while examining the influence of image-quality factors on model performance.Methods: A total of 261 pediatric high-grade glioma MRI datasets from a publicly available cohort were analyzed, each comprising four co-registered sequences (T1N, T1C, T2W, T2F). Two different DLMs were evaluated for lesion segmentation with an MR image provided as input: (i) MedSAM which uses bounding box prompts, (ii) BiomedParse which uses the text prompt “brain tumor segmentation”. Lesion segmentation accuracy was quantified via Dice overlap for each MRI sequence against available reference annotations. To assess the influence of image quality, 27 image quality metrics were extracted and their associations with segmentation accuracy were evaluated using Pearson correlations.Results: MedSAM significantly outperformed BiomedParse in lesion segmentation across all MRI sequences, achieving mean Dice scores of 0.80-0.86 compared to 0.17-0.49. MedSAM performed similarly on all sequences, while BiomedParse performed significantly better on T2w images. Specific quality factors influenced each FM differently. BiomedParse performance was negatively correlated with higher order noise measures such as PSNR and SNR7 to SNR8, and positively correlated with entropy-based sharpness metrics. MedSAM performance correlated primarily with intensity heterogeneity measures including coefficient of variation and joint intensity variation.Conclusions: Deep learning segmentation models can be adapted for automated pediatric glioma delineation on MRI, but optimizing their performance will require accounting for image quality and acquisition differences to ensure clinical utility for childhood brain tumors.

Public Health, Equity & Care Delivery

Improving Bystander CPR Self-Efficacy in Under-resourced Communities Through Experiential Training of Aspiring URiM Health Care Trainees

Frydson, Ingrid; Huang, Hui; He, Zhulin; Fleites, Vanessa; Pakenham, Hannah; and Kamat, Pradip

Presenting Author: Ingrid Frydson, MD, FAAP (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 40

Abstract

Introduction:Racial, ethnic, and economic disparities persist in out-of-hospital cardiac arrest (OHCA) incidence and survival, with poorer outcomes in under-resourced communities and among racial and ethnic minority populations. Bystander cardiopulmonary resuscitation (CPR) is critical for survival, yet low self-efficacy in recognizing cardiac arrest and initiating CPR remains a major barrier to bystander response in these groups. Targeting education to undergraduate and graduate students from underrepresented backgrounds, who are interested in pursuing health care careers, offers a key opportunity to enhance bystander preparedness. Based on a needs assessment showing low confidence in CPR skills, we developed an experiential, skills-based workshop to improve learner self-efficacy, knowledge, and preparedness in cardiac arrest scenarios, while also empowering participants to share their skills at home and within underserved communities. Methods:Twenty-three undergraduate and post-baccalaureate students, from underrepresented backgrounds, interested in health care careers participated in a 90-minute experiential, skills-based CPR workshop incorporating hands-on manikin practice and structured feedback. Learner confidence and CPR knowledge were assessed pre-workshop, immediately post-workshop, and at 3 months. Confidence was measured using Likert-scale surveys, and knowledge was assessed using multiple-choice questions. Comparisons between pre vs post survey and pre vs 3-month were performed using Wilcoxon signed rank test and McNemar's Chi-squared test, as appropriate. Results:Eighteen participants completed post-workshop assessments. Self-efficacy across three domains—recognizing cardiac arrest, performing high-quality CPR, and functioning effectively as part of a team—improved significantly immediately after training and remained significantly higher at 3-month follow up (pre vs post and pre vs 3-month comparisons; all $p < 0.05$). Median CPR knowledge score, including ventilation and compression quality, also improved significantly (pre-test 60.0 [IQR 40.0, 80.0] vs. immediate post-test 100.0 [IQR 80.0, 100.0], $p = 0.003$). Learner reflections indicated high satisfaction with the workshop, increased interest in formal CPR certification, and perceived readiness to intervene as bystanders in community settings. Conclusion:Experiential CPR training for underrepresented, future health care trainees, boosts self-efficacy and knowledge, allowing them to act and spread skills in their communities, with the potential to improve bystander response and reduce OHCA disparities.

Gastrointestinal, Hepatic & Renal

Glycogen Storage Diseases Type X (GSDX): A New Case and Comprehensive Literature Review of Clinical, Biochemical, and Molecular Features

Furuta, Yutaka; Al-Jaberi, Rana; Tinker, Rory; Wang, Lijun; Xing, Changhong; Phillips, John; Li, Hong

Presenting Author: Yutaka Furuta, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 8

Abstract

Background: Glycogen storage disease type X (GSDX) is a rare autosomal recessive disorder caused by biallelic PGAM2 pathogenic variants, resulting in phosphoglycerate mutase (PGAM) deficiency. Due to its rareness, only individual case reports have been published. We aimed to compile all published cases to highlight the clinical, biochemical, and molecular features.Methods: A PubMed literature search was conducted in October 2025 to identify all relevant publications on GSDX. Eligible cases required either 1) confirmed biallelic PGAM2 variants classified as pathogenic or likely pathogenic, or 2) documented decreased PGAM enzyme activity in skeletal muscle with consistent clinical manifestations. Extracted variables included age at onset and diagnosis, sex, ethnicity, consanguinity, clinical symptoms, serum creatinine kinase (CK) levels, PGAM2 variants, PGAM enzyme activity, muscle biopsy findings, and lactate and ammonia levels in response to forearm ischemic testing. Our new patient with GSDX was also included.Results: Including our new patient, 27 individuals with GSDX were identified. African American were most frequent (11/27), followed by Italian (5/27), Pakistani (3/27), Iranian (2/27), Japanese (2/27), German (1/27), and unknown ancestry (3/27). Symptom onset was typically during adolescence, but ranged from infancy to late adulthood. Muscle symptoms were often absent when well, with normally or mildly elevated CK levels during asymptomatic periods. Symptoms were consistently triggered by brief strenuous activity without other specific precipitating factors. The most frequent reported PGAM2 pathogenic variant, c.233G>A (p.Trp78Ter) was identified in 11/27 individuals (41%), all of African American ancestry, supporting a founder effect. Although no clear genotype–phenotype correlation was defined, homozygosity for the p.Trp78Ter was associated with markedly reduced PGAM enzyme activity (2.1-6%), significantly earlier diagnosis, and higher CK levels. Four symptomatic heterozygous carriers with partial PGAM deficiency (22-50%). Conclusions: Our report represents the largest and most up-to-date case series of GSDX. Diagnosis relies on careful clinical assessment, supported by molecular testing. When only a single PGAM2 variant is identified despite strong clinical suspicion, further studies such as muscle biopsy with PGAM enzymatic analysis or advanced genomic testing including short- or long-read sequencing may be required. Our case series provides clinical, biochemical, and genotype-phenotype data to support diagnosis and genetic counseling.

Gastrointestinal, Hepatic & Renal

SEMMA Study: Stress Microbiome and Metabolome in Inflammatory Bowel Disease, A Preliminary Analysis of Longitudinal Psychological Data in Patients with IBD

Gaini, Meghana, MD; Reed, Bonney, PhD; Jones, Rheinallt, PhD; Kugathasan, Subra, MD

Presenting Author: Meghana Gaini, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 9

Abstract

Background: Inflammatory bowel disease (IBD) is a chronic, remitting and relapsing intestinal disease. Adolescent patients must manage chronic gut inflammation while also dealing with real life daily stressors. It is well described that patients with IBD have higher mental health needs including increased rates of anxiety and depression. This study aims to characterize daily perceived stress in adolescents with IBD and its relationship to illness and mental health symptoms. Methods: We are recruiting 20 subjects, ages 11-21, with active IBD at enrollment. Participants receive bi-weekly surveys, beginning with a 2-question stress screener that measures psychological and physical stress. If they screen positive, the screener is followed by the PHQ-4 to measure symptoms of anxiety and depression, Psychological Stress Experiences PROMIS Pediatric 8 Question Short form to measure perceived stress, and a checklist of IBD and non-IBD related stressors. To date, we have collected longitudinal data from 15 participants. For each participant, we calculated baseline levels of depressive symptoms, psychological stress, and the number of stressors endorsed. In addition, we calculated mean levels of depressive symptoms and psychological stress across enrollment. The relationship between symptoms of psychological stress and the total number of stressors endorsed was analyzed for participants with positive stress screeners at enrollment. This analysis was also conducted within individuals across time. Results: Over half the participants (8/15) screened positive for stress at enrollment. Within these individuals, PHQ 4 scores ranged from normal to moderate levels of anxiety and depressive symptoms and symptoms of psychological stress ranged from average to almost 2 standard deviations (SD) above the mean. Most participants (7/8) identified primarily non-disease related stressors. Analysis revealed no significant correlation between PROMIS T scores at enrollment and the number of perceived stressors ($r=0.10$, $p=0.81$). Similarly, there was no significant correlation between symptoms of psychological stress and the number of perceived stressors within individuals across time. Conclusions: Participants who reported stress at enrollment primarily cited non-IBD related factors such as exams, poor sleep, and family conflict. Although no direct correlation was identified between psychological stress and number of stressors, in part due to small sample size and survey limitations, the results highlight that children with IBD face significant daily challenges unrelated to their disease. Therefore, IBD providers should routinely screen patients for mental health issues and guide stress management to support their transition to well-functioning adults.

Gastrointestinal, Hepatic & Renal

TNF-a and IFN-y Disrupt Epithelial Cell Mitochondrial Function and GLUT1 Localization During Active Crohn's Disease

Geiculescu Irina*, MD1,2; Munasinghe, Sachith*, MD1; Maddipatla Sushma, MS1; Syed, Sana, MD3; Vasantha L Kolachala, PhD1; Matthews, Jason, PhD1; and Kugathasan, Subra, MD1,2.

Presenting Author: Irina Geiculescu, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 15B

Abstract

Background: While immune involvement is well recognized in Crohn's disease (CD), emerging evidence suggests metabolic pathways may contribute to CD pathogenesis. Previously published analyses of ileal CD-derived organoids revealed an association of the mevalonate pathway with active disease, implicating epithelial metabolic imbalance during inflammation. We investigated the effects of inflammatory cytokine exposure on ATP levels and examined the role of HMG-CoA reductase in this process. Methods: Ileal organoids established from CD patients at Children's Healthcare of Atlanta were cultured in high (25mM) or low glucose (5mM) media, stimulated with IFN-**γ** (1ng/mL), TNF-**α** (25ng/mL), and/or inhibitors for 24 hrs: Atorvastatin (10 μM), an HMG-CoA reductase inhibitor, Rotenone (250 nM), an inhibitor of the electron transport chain, and ST1326 (25 μM), a CPT-1 inhibitor of long chain fatty-acid transport into mitochondria. ATP levels were measured with CellTiter-Glo® 3D Cell Viability Assay. Levels of SLC2A1 mRNA encoding GLUT1 were assessed by ileal single-cell RNA-seq data. GLUT1 localization was observed in paraffin sections from ileal CD tissue and organoids by immunofluorescence microscopy. Results: Organoids co-stimulated with TNF-**α**/IFN-**γ** had significantly reduced ATP levels (Fig.1A). Levels of ATP were not affected by ST1326 (Fig.1C). Like TNF-**α**/IFN-**γ** stimulation, organoids cultured in low-glucose media (5 mM) showed a marked reduction in ATP production but didn't show evidence of cell death (Fig.1D–E). The largest decrease in ATP levels occurred with application with rotenone (Fig.1F). Interestingly, single-cell RNA-seq analysis indicated that SLC2A1 was upregulated in epithelial cells during active treatment-naïve CD (Fig.2A–B). However, GLUT1 accumulation was reduced at the apical surface in active CD mucosa, localizing primarily to cytoplasmic vesicles in the basolateral compartments within epithelial cells, structures not observed in the non-inflamed control tissue (Fig.2C). TNF-**α**/IFN-**γ** stimulation of organoids demonstrated similar patterns of disrupted GLUT1 localization. (Fig.2D) Notably, atorvastatin treatment blocked the TNF-**α**/IFN-**γ**-induced decreases in ATP levels. (Fig.2E) Conclusion: These results link mitochondrial dysfunction during inflammation with HMG-Co reductase activity and aberrant vesicular trafficking of GLUT1 to the apical plasma membrane. GLUT1 appears immobilized in perinuclear space , indicative of vesicles departing from the Golgi, suggesting the receptor's transport to the apical membrane is reduced during CD.

Neurodevelopment & Autism Interventions

Understanding the Implementation Challenges and Strategies of EarliPoint in an Integrated Primary Care Service Setting

Gonzalez Laca, Alexa ; Kim, Jun ; Ransom, Lyric ; Menon, Nina ; Demetri, Brooke and Kuhn, Jocelyn

Presenting Author: Alexa Gonzalez Laca, MPH (Clinical Research Coordinator)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 18

Abstract

Background: The specialized autism workforce has not sufficiently grown with the rapid increase in autism prevalence, resulting in widespread delays in diagnosis and treatment. EarliPoint has the potential to improve the efficiency of autism evaluations, thereby alleviating waitlists and facilitating earlier access to care. Initial feedback from early adopters suggests that barriers interfere with the promise of EarliPoint. As a result, we anticipate identifying challenges that are affecting its implementation at early adopter sites. This mixed-methods study aims to harness methods from implementation science to identify, develop, and apply strategies to maximize the positive impact of EarliPoint across one early adopter site. Methods: To characterize the autism service system at the study site, we recruited participants clustered by role (e.g., caregivers, clinicians, care coordination staff). Participants engaged in a 4-hour focus group that consisted of: i) sociodemographic and professional background survey, ii) a group process mapping exercise to document the processes, dynamics, and challenges of autism evaluation services utilizing EarliPoint, iii) Failure Mode and Effects Analysis (FMEA) for each challenge documented on the process map, and iv) consensus building around solutions to highest priority challenges using nominal group technique. Highest priority challenges were determined as part of the FMEA exercise: participants assigned Severity (1=Insignificant, 5=Critical), Probability (1=Nearly Impossible, 5= Certain), and Detection (1=Certainly Detected, 5=Impossible) ratings for each identified challenge, which were multiplied together to create a Risk Priority Number (RPN). Results: Preliminary findings from focus groups with administrators, clinicians, and care coordination staff (n=8) indicated several barriers and workflow challenges. Highest priority challenges included the lack of diversity among the characters depicted in the EarliPoint videos (m RPN rating=31.5), no available pathway to refer more complex cases to for specialty care (m RPN rating=24.8), and the limited designated assessment space for administration and storage (m RPN rating=21.3). Conclusion: These findings highlight that there are important implementation challenges that have arisen for early EarliPoint adopter site stakeholders. When introducing new technology to systems of care, it is important to iteratively refine workflows and processes to maximize the positive impact of technology for families, providers, and systems.

Public Health, Equity & Care Delivery

Pediatric Bag Mask Ventilation Teaching Using Artificial Intelligence for Simulation and Debriefing: Feasibility Study

Gorbatkin, Jenna; Holmes, Sherita; Norris, Kimberly; Langham, Joseph; Okonye, Jeffrey; Patel, Sajani; Young, Deborah; Jergel, Andrew; Ottolin, Tommy; Liu, Songyuan; Sun, Jinghan; Zhou, Junyi; and Hwu, Ruth

Presenting Author: Jenna Gorbatkin, MD, MSEd (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 36

Abstract

BackgroundAlternative approaches are needed to teach lifesaving skills such as bag mask ventilation (BMV) when resources for traditional in-person methods are limited. This study aimed to evaluate the feasibility of using an artificial intelligence (AI) chatbot for simulation and debriefing with pediatric residents to improve BMV skills. Our secondary aim was to evaluate the acceptability of this method with learners. MethodsA prospective educational feasibility study was conducted with 20 pediatric residents. Participants completed an AI chatbot driven simulation and debriefing case on BMV skills. Pre and post-intervention BMV skills were assessed using a 15-point modified version of a validated checklist. Residents completed a post-intervention survey assessing satisfaction and preferred learning style. Descriptive statistics including means with standard deviations (SD) of pre and post-intervention scores (range 0-15) and mean score change (95% confidence interval) were calculated. Individual checklist components were summarized using counts and percentages. Mean resident satisfaction and preferred learning style were calculated based on 5-point Likert scales. ResultsThe average BMV skills score improved from 7.3 (SD=4.33) to 11.4 (SD=2.76) on 15-point checklist from pre to post intervention (P=0.001). Individual checklist items with improvement from pre to post were all within the subcategory of management of the airway: universal maneuvers to open airway (P<0.001), choosing correct size oropharyngeal airway (OPA) or nasopharyngeal airway (NPA) (P=0.018), inserting OPA or NPA correctly (P<0.001), and correct positioning of the head (P<0.001). 85% of participants were satisfied or very satisfied with the AI module overall for simulation and debriefing, and 65% were satisfied or very satisfied with the AI module specifically for BMV skills. Only 25% of participants agreed or strongly agreed they would prefer the AI module for simulation and debriefing over in-person simulation and debriefing. ConclusionsThe use of AI for simulation and debriefing is a feasible method for improving BMV skills of pediatric residents overall but was more effective for a subset of BMV skills. The use of AI for simulation and debriefing is an acceptable method by users; however, it was not preferred over in-person simulation and debriefing.

Public Health, Equity & Care Delivery

Complement Inhibition in Pediatrics: A Comprehensive Single-Center Analysis

Graciaa, Sara; Dave, Ronak; Pickett, Logan; Yee, Marianne; Fasano, Ross; Dean, Gabrielle; Verma, Sumit; George, Roshan; Jernigan, Stephanie; Greenbaum, Larry; Briones, Michael; Schoettler, Michelle; and Chonat, Satheesh

Presenting Author: Sara Graciaa, MSN (Pediatric Nurse Practitioner)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 41

Abstract

Background: Complement inhibition (Ci) has revolutionized outcomes in disorders, such as paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), by improving prognosis and quality of life. However, Ci increases susceptibility to infection, despite immunization and prophylactic antibiotics. While infection risk with encapsulated bacteria is well documented, the true extent of this risk is not well characterized in children. Methods: In this IRB-approved, retrospective study, we identified patients treated with Ci at CHOA from 2010 to 2025. Infections were identified via positive culture, and data were reviewed from 1st dose of Ci to 6 months following last dose or date of death. Infection rate was calculated as # infections/days at risk. Results:Infection rates were assessed in 96 patients over 227 patient years. Median age at first Ci dose was 15 years and 52% were male. Ci indications included a cohort of immune competent children: aHUS (n=23), C3G(n=6), STEC-HUS (n=4), classic PNH (n= 9), and SARS-CoV-2 (n=1). The second cohort included patients who received additional immune suppression or had asplenia, including SCD hyperhemolysis (n=29), autoimmune hemolytic anemia (AIHA) (n=4), PNH with bone marrow failure (BMF) (n= 12), refractory MG (n=3), and other (n=5). Among the immune competent cohort (n= 43), only 1 patient with STEC-HUS developed an infection, pseudomonas bacteremia. Nine immunocompromised patients developed significant infection(s), including bacteremia (n=5), candidemia (n= 1), invasive mold (n= 1), and pneumonia (n=2). Cumulative incidence of infection was significantly higher in the immunocompromised patients (1-yr estimate 12% vs 2%, p=0.008) and they had significantly higher infection rate (p=0.016). There were ten deaths: 9 immunocompromised and 1 immune competent patient/s. Two deaths were attributed to encapsulated bacterial infection, but both infections were present prior to Ci. Conclusion: This comprehensive institutional review demonstrates the favorable safety profile of Ci in pediatrics. Notably, no cases of meningitis were linked to Ci and duration of Ci was not associated with increased infection. There were significantly more infections in the immunocompromised cohort, but there may be additional confounding factors. These data emphasize importance of ongoing vigilance and education regarding infection risk and early symptom recognition.

Oncology & Cellular Therapy

Association Between BMI at Diagnosis and Development of Anorexia in Children Undergoing Treatment for Acute Lymphoblastic Leukemia

Haber, Rebecca DO; Lee, Katie MPH; Stevenson, Jason MD MSCR; Hawk, Ashleigh MPH; Hunt, Amy; Khanna, Anjali MBBS, MPH; Park, Yoonseo; Castellino, Sharon M. MD, MSc; and Miller, Tamara P. MD, MSCE

Presenting Author: Rebecca Haber, BA, DO (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 26

Abstract

Background: Despite high survival rates, children with acute lymphoblastic leukemia (ALL) commonly experience treatment-related adverse events (AEs), including anorexia that can impact quality of life. Nutritional status at diagnosis, commonly measured by body mass index (BMI), may influence treatment tolerance, but the relationship between BMI and development of anorexia remains poorly defined. Existing literature describes associations between extremes of BMI and survival outcomes in ALL, but studies investigating anorexia in relation to BMI are limited. Clarifying this association may help identify high-risk patients and guide surveillance. Methods: This retrospective cohort study included children aged 1-21 years treated for ALL at Children's Healthcare of Atlanta from 2010-2022. BMI at time of ALL diagnosis was calculated and categorized per CDC guidelines. Grade 2+ clinically-treated anorexia (CT-anorexia), defined per Common Terminology Criteria for Adverse Events (CTCAE) v5.0 definitions, was manually abstracted. The primary outcome was development of CT-anorexia at least once during therapy. Unadjusted logistic regression models evaluated association between BMI and CT-anorexia. Results: Of 290 patients in the cohort, 190 (66%) were underweight prior to chemotherapy, 70 (24%) were normal weight, 22 (7%) were overweight, and 8 (3%) were obese. Overall, 117 (40%) had 1+ AE of CT-anorexia. Among these, 67 (57%) patients were underweight, 35 (30%) were normal weight, 13 (11%) were overweight, and 2 (1.7%) were obese. Compared to normal weight patients, underweight patients had significantly lower odds of developing CT-anorexia (OR=0.54, 95% CI: 0.31–0.94, p = 0.028). Odds did not differ significantly for overweight (OR=1.44, 95% CI: 0.55–3.91) or obese patients (OR=0.33, 95% CI: 0.05–1.56). Conclusion: Anorexia was common, with 40% of patients requiring intervention during ALL therapy. Most patients who developed CT-anorexia were underweight, reflecting the underlying BMI distribution of the study cohort. However, the relative odds of developing CT- anorexia was significantly lower for underweight compared to normal weight patients. These findings suggest anorexia is not limited to underweight patients and may occur among children with normal or higher baseline BMI. Future analyses will evaluate demographic factors and characterize the development of CT-anorexia by specific chemotherapy course to identify high risk populations and periods.

Public Health, Equity & Care Delivery

Lockboxes for Little Lives: Implementing Firearm Screening for Families of Toddlers in the Pediatric Inpatient Setting

Heather Hirsch, Sofia Chaudhary, Kiesha Fraser Doh, Amanda Kuhn, Kelly Liu, Sajani Patel, Nitya Ramalingam, Jaqueline Solis Solis, Shelby Thomas, Heather Xiao, Julia Yarahuan, Melissa Popkin, Lori Singleton

Presenting Author: Heather Hirsch, MD, MPH (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 42

Abstract

Firearms currently surpass motor vehicle collisions as the leading cause of death for children and youth in the United States. Families often underestimate their child's access to firearms and fail to recognize that children as young as two years old may have enough strength to pull the trigger. Conversely, storing firearms locked or unloaded is approximately 70% protective. Provider guidance and safety device provision improves firearm secure storage rates, yet fewer than 3% of inpatient pediatricians discuss firearm safety despite two-thirds of parents, including 58% of firearm-owning parents, supporting such conversations. We implemented a quality improvement initiative to address gaps in inpatient firearm screening. Our primary aim was to increase documentation of household firearm access among Pediatric Hospital Medicine (PHM) patients aged 2-5 years from <2% to 40% by December 2025. The secondary aim was to distribute handgun lockboxes to 80% of families reporting unsecured firearms. The global aim was to increase parental knowledge of secure firearm storage strategies and reduce firearm injuries in toddlers. The project involved pediatric residents, PHM fellows, PHM faculty, and social workers. We collected baseline data through a randomized review of 20 charts per month from June 2024 to November 2024. A key driver diagram was completed to identify potential interventions including a screening tool embedded within the Electronic Medical Record system and educational sessions with physicians and social workers. The hospital's parental advisory committee provided input on screening questions. From June 2025 to December 2025, we conducted plan-do-study-act cycles. The monthly percentage of patients with documentation of firearm access was tracked using an annotated run chart. Upon discharge, families who endorsed access to firearms received written firearm safety guidance and watched a secure firearm storage video. Families who owned an unsecured firearm were educated on lockbox use by a social worker and offered a lockbox. We plan to contact families regarding lockbox usage. To date, we have completed 6 months of post-intervention data and have found an average screening rate of 11.4%, improved from a baseline screening rate of 1.7%. We've distributed 26 lockboxes with a lockbox distribution rate of 70% for eligible families.

Neurodevelopment & Autism Interventions

Establishing Developmentally Appropriate Benchmarks for Social Communication for Non-Autistic Preschoolers

Johnson, Paige; Edmier, Kathleen; Naresh, Aparna; Keleher, Courtney; Bien, Elena; Lampert, Erica; Argueta, Tracy; Fuhrmeister, Sally; Edwards, Laura; and Yosick, Rachel

Presenting Author: Paige Johnson, MS (PhD Student)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16501 and 16502
Poster Number: 88

Abstract

Background: Preschool children with autism often demonstrate fewer prosocial behaviors (e.g., initiations and responses) during peer interactions in inclusive classrooms. Social communication interventions for autistic individuals can support meaningful relationships, mental health, and later academic success. While commonly used skills assessments compare individuals against established criteria, they lack benchmarks for social communication frequencies at specific developmental ages. Therefore, this study aims to identify benchmarks from non-autistic preschoolers to guide developmentally appropriate social and language intervention goals for children on the autism spectrum. We hypothesize that the overall frequency of social communication will vary by age group and that the types of communication will change by age as well. Methods: In the current study, preschoolers' social interactions in structured and unstructured activities in their classroom setting were video recorded. Recorded videos were then hand-coded for social communication behaviors by members of the research team. Two trained coders independently recorded the frequency of vocal verbal operants (VVOs): mands (requests), tacts (labels), sequelics (two-turn exchanges), and conversational units (three-turn exchanges). Interobserver agreement was calculated, and discrepancies were resolved through discussion. Results: Study participants include 19 non-autistic children with and without other neurodevelopmental diagnoses, ages 3–5, who were enrolled in an inclusive preschool classroom. This project includes the preliminary coding analysis of four 4-year-old children across two videos and activities. Across 10-minute sessions, children emitted an average of 30.5 VVOs (range 15–57), including an average of 9.5 mands (range 1–19), 8.75 tacts (range 5–16), 2.75 sequelics (range 0–5), and 9.5 conversational units (range 1–18). Conclusions: The preliminary data suggest that four-year-olds typically emit about 30.5 verbal operants during free play with peers. Additional coding of all 19 participants will help establish developmentally appropriate benchmarks for frequency and variety of social communication behaviors in non-autistic preschool children.

Gastrointestinal, Hepatic & Renal

Imaging Phenotypes of Pediatric MASLD Using Quantitative MRI

Jordan, Gregory A.; Sun, Binjian; Krupinski, Elizabeth; Alazraki, Adina; and Khanna, Geetika

Presenting Author: Gregory Jordan, MD (Junior Faculty)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 10

Abstract

Background: Metabolic dysfunction associated-steatotic liver disease (MASLD) is now the most common cause of chronic liver disease in children, paralleling the rise in pediatric obesity. Early identification is critical, as fibrosis stage is the strongest predictor of long-term outcomes. The purpose of our study was to determine the impact of hepatic steatosis on liver stiffness in a pediatric cohort. Methods: This was a retrospective, IRB approved study. The radiology information system was searched to identify patients younger than 20 years who underwent liver MRI for fat estimation and MR elastography (MRE) for stiffness between 2017-2025. MRI was performed on the same day in all cases, and all scans were acquired on a 1.5T scanner. Patients with secondary causes of liver disease, including congenital heart disease, hepatotoxic chemotherapy/medication, or genetic liver conditions were excluded. Liver steatosis was estimated by segmentation of the liver using proton density fat fraction(PDFF). Mean liver stiffness was obtained from MRE maps by an imaging scientist blinded to all other data, using freehand regions of interest placed over the mid-liver on the autogenerated 95% confidence stiffness map. Data were summarized with descriptive statistics, and the Pearson correlation coefficient was calculated to evaluate the relationship between fat fraction and liver stiffness.Results: Our cohort included 82 patients with a mean age of 13.6 years. By phenotype, 14 patients had normal PDFF and stiffness, 46 had elevated PDFF and normal stiffness, 16 had elevated PDFF and elevated stiffness, and none had normal PDFF and high stiffness. The remaining 6 patients either had incomplete PDFF or MRE imaging. Mean PDFF was 14.5% ± 8.4 (range 1.4-37.6) and mean MRE was 2.7 ± 0.74 kPa (range 1.6-5.7). Elevated PDFF (>5%) was observed in 85% of patients, and elevated stiffness (>2.8 kPa) in 30%. PDFF and MRE were not significantly correlated ($r = 0.04$; $z = 0.31$, $p = 0.76$). Conclusions: Hepatic fat alone does not significantly influence liver stiffness in pediatric MASLD. Most children with elevated PDFF show normal stiffness in our cohort, suggesting steatosis and fibrosis develop independently. MRI-based fat/stiffness phenotyping can offer a noninvasive approach for early characterization and risk stratification in pediatric MASLD.

Sickle Cell Disease & Transfusion Science

Moving from Caregiver to Supporter: Exploring Parenting Practices and Healthcare Transition Knowledge, Needs, and Concerns Among Parents/Caregivers of Adolescents and Young Adults with Sickle Cell Disease (SCD)

Kalter, David; Tang, Amy; Williams, Justin; Johnson, Shamona; and Christie, Trinity

Presenting Author: David Kalter, PsyD (Postdoctoral Fellow)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16503 and 16504
Poster Number: 101

Abstract

Background: The transition from pediatric to adult healthcare can pose significant challenges for adolescents and young adults (AYAs) with sickle cell disease (SCD). This developmental shift is compounded by disease-specific vulnerabilities, leading to increased morbidity, mortality, and healthcare utilization. Parents and caregivers play a critical role in promoting self-management and transition readiness, yet little empirical work has characterized their transition-related knowledge, parenting practices, emotional experiences, or perceived support needs.Objective: This multisite quality improvement (QI) initiative aims to characterize caregiver knowledge, preparedness, emotional responses, and instructional practices related to the transition to adult SCD care. Findings will inform the development of targeted, caregiver-centered educational resources integrated across pediatric, adult, and community SCD care systems.Methods: Parents/caregivers of AYAs with SCD (ages 16–35) receiving care across four Georgia health systems and the Sickle Cell Foundation of Georgia will complete a questionnaire assessing transition awareness, healthcare navigation skills, disease-management teaching, concerns about transfer, and preferences for transition-related information. Approximately 150 caregivers will be approached across pediatric, adult, and community sites. Data will be collected through secure platforms (REDCap, Qualtrics, or encrypted databases). This stage of the project will focus on identification of knowledge gaps, emotional themes, and unmet educational needs.Planned Intervention: Questionnaire findings will guide the creation of caregiver-focused educational materials (e.g., handouts, videos, resource guides) on transition to adult-based care. In 2026-2027, materials will be piloted for acceptability, relevance, and perceived utility.Implications: By centering caregiver perspectives, this QI project seeks to enhance transition readiness and promote the adaptive shift from “healthcare manager” to “healthcare supporter.” Limitations: This QI project is constrained by a geographically limited and convenience sampling, which may limit generalizability. Findings rely on caregiver self-report, introducing potential recall and social desirability biases. The questionnaires are newly developed and lack established psychometric validation, which may affect interpretation of results.

Pulmonary, Respiratory Support & Cystic Fibrosis

SARS-CoV-2 Priming Exacerbates Influenza Severity and Mortality

Meenakshi Kar, Shilpi Jain, Katharine Floyd, Stephanie L. Foster, Jacob Vander Velden, Jacob Kohlmeier, Mehul S. Suthar

Presenting Author: Meenakshi Kar, PhD (Postdoctoral Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 31

Abstract

Respiratory virus co-infections, particularly with SARS-CoV-2 and influenza, have been linked to worsened disease outcomes in humans, including increased hospitalization, mechanical ventilation, and mortality. However, the impact of prior SARS-CoV-2 infection on subsequent influenza virus pathogenesis remains incompletely defined. Using a B.1.351 SARS-CoV-2 mouse model, we investigated the effect of sequential SARS-CoV-2 and influenza infection at varying intervals post-SARS-CoV-2 clearance (7, 14, 21, 28, and 180 days). Lung viral loads, histopathology, innate immune cell recruitment, cytokine production, and alveolar macrophage activation were assessed. We found that prior SARS-CoV-2 infection creates a transient window of immune dysregulation during recovery that predisposes to worsened influenza outcomes. Mice infected with influenza at later recovery timepoints (~28 days) exhibited exacerbated disease and impaired neutrophil and eosinophil recruitment compared to influenza-only controls. This heightened susceptibility was associated with delayed resolution of inflammatory gene expression programs and sustained MHC-I/II and CD86 expression in the alveolar macrophages. These findings provide mechanistic insight into clinical reports of severe disease in SARS-CoV-2/influenza co-infections and highlight the importance of timing in mitigating risk.

Neurodevelopment & Autism Interventions

Evaluation of Georgia's ECHO Autism: Primary Care Early Diagnosis Training Model

Kim, Justin; Gonzalez Laca, Alexa; Ransom, Lyric; Zubler, Jennifer; Schwartz, Allison; Nelson, Natasha; Booth, Chris; Constantino, John; and Kuhn, Jocelyn

Presenting Author: Justin Kim, BS (Predoctoral Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 19

Abstract

Background: Early identification of autism is a critical entry point to accessing evidence-based interventions and services that can improve a child's developmental outcomes. Although autism can be reliably diagnosed at 15 months-old, the average age of diagnosis in the U.S. is > 4 years-old. Delays in diagnosis reflect a growing mismatch between the number of children needing evaluation and the availability of qualified clinicians. The ECHO Autism: Early Diagnosis Model is an evidence-based program designed to address this gap by training primary care providers (PCPs) to screen and diagnose children aged 14–48 months and provide longitudinal care for families. Methods: For the year-long program, we recruited 19 PCPs from seven different counties across Georgia. PCPs participated in a two-day in-person training session for administering the Screening Tool for Autism in Toddlers and Young Children (STAT). They achieved reliability in administering the STAT instrument and attended virtual ECHO sessions twice a month. At each session, a PCP presented a case, and our hub team of interdisciplinary autism experts provided guidance on the PCP's diagnostic process, decision, and recommendations. A self-report survey was used to measure PCP self-efficacy in autism evaluations and knowledge of autism-related topics at baseline, and survey administration will be repeated in February 2026. Survey items were rated on a five-point Likert scale (0 = no confidence; 4 = high confidence). Results: Pre-program survey responses were completed by 18 PCPs. Baseline self-efficacy varied across autism-related clinical domains. PCPs reported the highest self-efficacy in learning outcomes related to autism identification (M=2.85) and in managing constipation in autistic children (M=2.50). In contrast, providers reported descriptively lower confidence in managing sleep-related concerns (M=1.94) and nutritional deficiencies (M=1.97), which are common co- occurring conditions in autistic children. Moderate levels of self-efficacy were observed for supporting families (M=2.13) and managing ADHD, anxiety, and interfering behaviors with autism (M=2.12). Conclusion: The baseline self-efficacy ratings provide an initial profile of the PCPs' confidence and will inform evaluation of the training model. Post-program survey data will be collected before the conference presentation date and analyzed to examine changes in PCP self-efficacy following program participation.

Reproductive, Adolescent & Women's Health

Mapping the Evidence on GLP-1 Receptor Agonists for the Treatment and Prophylaxis of Metabolic Adverse Effects of Antipsychotics in Youth With Psychiatric and Neurodevelopmental Disorders: A Scoping Review

Klein, Jessica; Susskind, Bradley; Massaro, Alexandra; Luu, Skylar; Bempong, Phillip; Rutkowski, Eric

Presenting Author: Jessica Klein, BS (MD Student)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 44

Abstract

Background:Anti-psychotics are utilized in the management of psychiatric and neurodevelopmental disorders in children and adolescents. However, they can induce notable metabolic disturbances, including weight gain, insulin resistance, and dyslipidemia, which may persist into adulthood and increase cardio-metabolic risk. Current pediatric interventions intended to mitigate these metabolic adverse effects, including metformin and lifestyle changes, are marginally effective, highlighting the need to investigate alternative approaches, such as GLP-1 (glucagon-like peptide-1) receptor agonists. GLP-1 receptor agonists are effective in managing obesity and type 2 diabetes in adults and children, suggesting their potential to reduce anti-psychotic-related metabolic syndromes in youth. This scoping review examines existing literature concerning the application of GLP-1 receptor agonists in pediatric anti-psychotic treatment.Methods:A PRISMA-ScR-compliant search was conducted on December 10, 2025, using Medical Subject Headings and database-specific terminology across PubMed, Scopus, EMBASE, PsycINFO, and CINAHL. Records were uploaded to JBI SUMARI, where two reviewers independently screened titles and abstracts, followed by full-text review with adjudication of conflicts. Eligible studies included participants ≤18 years treated with anti-psychotics who received GLP-1 receptor agonists for anti-psychotic-related metabolic adverse effects, with outcomes including BMI, or glycemic control.Results:Fifty-seven unique records were identified; forty-nine were excluded following title and abstract screening. Eight full texts were reviewed. Four trials examined GLP-1 receptor agonists for obesity and metabolic syndromes related to anti-psychotic therapy, however, all enrolled exclusively adult populations (median age in the fifth decade). The remaining four articles were reviews or meta-analyses of adult cohorts. Three papers addressing GLP-1 receptor agonists for pediatric diabetes and obesity were identified, however, they were excluded during title and abstract screening, as they did not address anti-psychotic-induced metabolic syndromes. No study investigated GLP-1 receptor agonist therapy for antipsychotic-induced metabolic effects in children or adolescents. While findings from general pediatric obesity studies may offer indirect insight, there remains a critical need for investigating GLP-1 therapy in youth receiving anti-psychotics.Conclusions:The absence of evidence on GLP-1 receptor agonists for addressing anti-psychotic-induced metabolic complications in children demonstrates a critical gap. This review highlights the need for anti-psychotic specific pediatric studies guided by existing adult and pediatric data.

Gastrointestinal, Hepatic & Renal

Chronic Feeding Disorder in Patients with Eosinophilic Esophagitis: A Descriptive Study of a Novel Population

Kohley, Annalyse; Oh, Teresa; Proctor, Kaitlin; McElhanon, Barbara

Presenting Author: Annalyse Kohley, BA, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 11

Abstract

Background: Pediatric feeding disorder (PFD) encompasses all feeding difficulties that prevent a child from obtaining adequate nutrition by mouth in a manner that is appropriate for their age and skill level. Patients with eosinophilic esophagitis (EoE) can develop PFD due to mechanistic differences in the esophagus secondary to uncontrolled disease or through learned maladaptive behaviors. Our study aims to address the limited data on the success of intensive feeding therapy for patients with PFD and EoE and to describe a novel group of patients demographically with EoE and PFD. We hypothesize that the average percentage of treatment goals met at discharge for PFD patients with EoE equals the average percentage of goals met for PFD patients not diagnosed with EoE. Methods: This pilot retrospective cohort study evaluates patients with EoE and PFD who underwent treatment with Children's Healthcare of Atlanta Multidisciplinary Intensive Feeding Program from 2022-2025. Results: 29 Patients were analyzed. The population was 76% male, 62% African American, 31% White, 3% Asian, and 3% Other. Age range between 1-18 with average age 5.38±3.83 years. 58.6% had autism and 63% had some form of developmental delay. Per RD standardized evaluation, 73% of patients had nutritional deficiencies and 35% had malnutrition. Feeding concerns started at an average age of 15.71±6.70 months. Average age at time of EoE diagnosis was 48.63±40.43 months with minimum age 5 months and maximum age 15 years. Caregivers reported history of the following 46% gagging, 50% predominance of liquid or puree diet, and 54% food refusal. Average percentage of goals met was 96.1±5.09 comparable to overall program percentage of goals met >90%. In this EoE cohort, portion of nutrition provided by formula before treatment was 31.33±37.76 versus 23.08±23.23 after intervention. Prior to intervention, 11 patients required ≥35% of nutrition from formula compared to 22 patients required ≤35% formula after intervention. During follow-up approximately 1 month after treatment, 90.9% maintained behaviors and 83.8% maintained use of protocol meals. Conclusions: PFD patients with EoE have similar outcomes compared to PFD patients not diagnosed with EoE in intensive feeding program. Further investigation will be completed with enlarged sample size.

Neurodevelopment & Autism Interventions

Anxiety-Related Differences in the Assessment of Autism Symptomatology

Kolios, Alex; Walum, Hasse and Klaiman, Cheryl

Presenting Author: Alexander Kolios, BS (Predoctoral Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 20

Abstract

Background: Symptomatology overlap in anxiety and autism makes distinction challenging for clinicians and caregivers, especially in co-occurring cases. While adapted anxiety measures have been developed to address these challenges in anxiety identification for Autistic individuals, the differential autism diagnostic process in anxious individuals is still largely unexplored. Methods: Caregiver-evaluation analyses included 7,292 dyads, chosen based on completion of a Social Communication Questionnaire (SCQ; parent-report) and a Child Behavior Checklist (CBCL). All children had an autism diagnosis and were 7 to 12 years old. All dyads were recruited through the SPARK cohort. Factor analytic methods were employed to compare the psychometric structure of the SCQ scores between Autistic children with typical and elevated levels of anxiety symptoms. Results: An exploratory factor analysis on the SCQ questions revealed a five-factor model, each aligning with well-known constructs of autism-symptomatology (Reciprocity, Social Communication (SCI), Restricted and Repetitive Behaviors (RRB), Repetitive Speech, and Play). Measurement invariance testing of the SCQ structure between children with typical and elevated levels of anxiety symptoms was performed by comparing a configural model (where model parameters were free to vary between anxiety groups) to a scalar model where factor thresholds, loadings, and intercepts were constrained to be equivalent across groups. This comparison, according to standard fit criteria (CFI: -Δ0.01), indicated that the factor structure of SCQ items does not differ substantially between anxiety groups. Latent factor means revealed significantly higher scores for the elevated anxiety group on the Repetitive Speech (0.576, SE=0.205, p=0.005) and RRB factors (0.576, SE=0.205, p<0.001), whereas the typical anxiety group had significantly higher SCI (-0.146, SE=0.023, p<0.001) and Play factor scores (-0.277, SE=0.052, p<0.001). Conclusion: In adolescent-aged Autistic children, the factor structure of the SCQ items does not seem to differ between individuals with typical or elevated levels of anxiety. However, the groups had significantly different latent factor mean scores. Specifically, they suggest higher parent-reported RRB-related symptomatology in the elevated anxiety group, and higher SCI symptomatology in the typical anxiety group. Thus, differences in SCQ scoring may be due to the parents' perceived presentation of the child, instead of the measures' inconsistencies across groups.

Infectious Disease & Immune Dysregulation

Effects of Nirmatrelvir-Resistant Mutations in SARS-CoV-2 Mpro on Activity and Drug Efficacy

Lee, Rachel; Neilsen, Grace; and Sarafianos, Stefan

Presenting Author: Rachel Lee, BS (Research Specialist)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16403 and 16404
Poster Number: 74

Abstract

Background: Severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) is the causative agent of the Coronavirus Disease 2019 (COVID-19) pandemic. The main protease (Mpro) of SARS-CoV-2 is a potential drug target for COVID-19 treatments due to its critical role in the SARS-CoV-2 infectious cycle and dissimilarity to human proteases, and it is the target of Paxlovid, a Pfizer antiviral that includes the Mpro inhibitor nirmatrelvir (NIR). The E166V mutation in Mpro was found in patients with repeated and prolonged treatment with Paxlovid. E166V results in high NIR resistance but comes with a fitness cost due to distortion of the active site and loss of dimerization, which is necessary for proteolytic activity. The L141T mutation was identified in SARS-CoV to stabilize the active site of the enzyme and increase activity. I determined whether the introduction of the L141T mutation would increase dimerization and thus the enzymatic activity of the Omicron (BA.1) E166V Mpro without decreasing NIR resistance.Methods: A fluorescence-based assay was used to determine the relative kinetic parameters of the BA.1 Wildtype, E166V, and L141T/E166V constructs, and the half-maximal inhibitory concentrations (IC50) for the inhibition of these constructs by NIR and GC376. GC376 is a protease inhibitor used against the feline infectious peritonitis virus (FIPV) and also inhibits the SARS-CoV-2 Mpro. Size Exclusion Chromatography with Multi-Angle Light Scattering (SEC-MALS) was used to characterize the monomer-dimer equilibrium for the three constructs in the absence and presence of NIR and GC376.Results: The L141T mutation decreases proteolytic activity of the BA.1 E166V Mpro and partially rescues the dimerization lost from the E166V mutation. The L141T/E166V construct still demonstrates significant NIR resistance, but a decrease from that of the E166V construct. Conclusion: While introducing the L141T mutation increased dimerization, it did not fully rescue activity in the L141T/E166V double mutant. Additionally, the L141T mutation decreased the NIR resistance for the E166V mutant. Given the rapid mutation rate of coronaviruses and other ssRNA viruses, it is important to anticipate potential drug-resistance mutations to ensure that there is no over-reliance on a single antiviral drug, which can provide crucial information for designing future inhibitors.

Cardiac Bioengineering & Platforms

Outcomes of Allosensitized Pediatric Patients Supported by Ventricular Assist Devices: A Single-Center Retrospective Study

Mats Steffi Jennifer Masilamani, MD1, Gina Patel, MD2, Simran Jandu, MD1, Chad Mao, MD1, William T. Mahle, MD1, Arene Butto, MD1 2*

Presenting Author: Mats Steffi Jennifer Masilamani, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 3

Abstract

BackgroundChildren requiring support of ventricular assist devices (VADs) often have concurrent end-organ dysfunction, necessitating interventions such as blood product transfusions, thereby increasing their risk of allosensitization. This study aims to assess trends in calculated panel reactive antibody (cPRA) levels in children on VAD support, including response to desensitization strategies, and examine clinical outcomes. Methods We retrospectively reviewed patients ≤ 21 years of age who were supported with VADs for ≥ 30 days at our institution between 1/1/2019 and 6/30/25. Allosensitization was defined using an institutional threshold of ≥8000 MFI and cPRA >50% was classified as high allosensitization. Results Among 83 patients who underwent VAD implantation, 32 (38.5%) met inclusion criteria. Table 1 summarizes baseline characteristics. Fourteen patients (44%) had cPRA = 0 using institutional thresholds. Figure 1 demonstrates cPRA trends from time points preceding VAD through heart transplant (HT) for the remaining 18 patients. Among seven patients with cPRA ≥50% who underwent desensitization therapy, cPRA decreased to <10% in four. One patient continues to receive desensitization therapy. Conclusion Over half of patients were allosensitized following VAD implantation, but their cPRA declined to <50% in 83% prior to HT. Over half of highly allosensitized patients responded to desensitization, highlighting its potential efficacy. Because several patients did not develop allosensitization, targeted monitoring and individualized intervention strategies may optimize HT readiness.

Oncology & Cellular Therapy

Health Related Quality of Life of Pediatric Brain Tumor Survivors with a History of Hydrocephalus

Mekonnen, Rebekah, BS; Janss, Anna, MD, PhD; Mazewski, Claire, MD; and Ingerski, Lisa, PhD, ABPP.

Presenting Author: Rebekah Mekonnen, BS (Predoctoral Fellow)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16403 and 16404
Poster Number: 80

Abstract

Objective: Hydrocephalus occurs in up to 50% of pediatric brain tumor (PBT) patients and may negatively impact long-term health-related quality of life (HRQoL) and neurocognitive outcomes. While various treatment modalities are now available to mitigate the condition (e.g. ventriculoperitoneal [VP] shunt, external ventricular drain [EVD], ventriculostomy), research regarding the impact of hydrocephalus treatment (i.e., VP shunt) on school functioning in PBT survivors is scarce. The current study aims are to describe HRQoL of PBT survivors with a history of hydrocephalus and to compare sequelae by treatment type and tumor location (e.g., posterior fossa, cerebellum, suprasellar). It was hypothesized that those with a VP shunt would experience greater HRQoL deficits. Methods: Data were extracted from a larger retrospective study investigating HRQoL in PBT survivors. Parents completed – the Pediatric Quality of Life Inventory (PedsQL), including the school functioning subscale as part of standard clinical care. Results: HRQOL data was available for 67 PBT survivors (53.7% female, 76.1% white). Multivariate analyses were conducted relative to tumor location (e.g. posterior fossa [26.9%], cerebellum [19.4%], suprasellar [14.9%], 4th ventricle [9.0%], and other [29.9%]). Averages in the VP shunt and "other" treatment groups for both total scaled HRQoL scores (67.0±20.4, 76.8±18.6, respectively) and school functioning scaled scores (62.2±19.3, 70.3±19.6, respectively) were below the normal average. Results suggested tumor location ($p = 0.030$) and VP shunt placement ($p = 0.019$) significantly contributed to parent-reported HRQoL ($R^2 = .158$, $F(4, 60) = 2.814$, $p = 0.033$). Insurance ($p = 0.075$), tumor location ($p = 0.092$), and VP shunt placement ($p = 0.080$) approach significance in parent-reported school scaled scores ($R^2 = 0.118$, $F(3, 63) = 2.821$, $p = 0.046$). Conclusion: VP shunt placement can contribute to HRQoL and school functioning outcomes in PBT survivors. Further research is needed to understand potential HRQoL and academic sequelae of hydrocephalus, subsequently minimize late-effects, and advance pediatric psychological well-being.

Public Health, Equity & Care Delivery

Pediatric Abscess Management: Who Gets Poked?

Menk, Paul; Shah, Lekha; Wetterhall, Madeline and Burger, Rebecca

Presenting Author: Paul Menk, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 37

Abstract

Background: Drainage procedures are traditionally considered the gold standard for managing cutaneous abscesses in the pediatric emergency department (PED). However, increasing evidence suggests that many patients are treated conservatively with antibiotics alone. While current clinical guidelines emphasize abscess size when determining the need for drainage, other clinical and demographic factors influencing this decision are not well defined. Objective: To identify clinical and demographic factors associated with whether pediatric patients with cutaneous abscesses undergo drainage procedures or are managed conservatively in the PED. Methods: We conducted a retrospective review of electronic medical records for patients aged 0–18 years evaluated in a high volume PED and its satellite PEDs from May 1 to August 31, 2024, with a discharge diagnosis of cutaneous abscess (ICD-10 L02). Exclusion criteria included multiple abscesses, underlying conditions (hidradenitis suppurativa, cancer, HIV, or immunodeficiency), or deep space abscesses. Patients who underwent drainage procedures were compared to those managed conservatively. Demographic and clinical variables were analyzed using chi-square tests, t-tests, and univariable regression. Results:
Of 409 patients identified, 324 met inclusion criteria; 181 (55%) underwent drainage and 143 (45%) were managed conservatively. Black patients were significantly less likely to receive a drainage procedure compared to white patients (OR 0.54 [0.34–0.87], p=0.01). Abscess size on physical exam did not differ between groups (2.96 vs 2.57 cm, p=0.18), though ultrasound measurements were larger in the procedure group (2.3 vs 1.71 cm, p=0.01). Abscesses located on the head/neck were less likely to be drained than those on extremities (OR 0.14 [0.05–0.33], p<0.001), and spontaneously draining abscesses were also less likely to undergo intervention (OR 0.5 [0.31–0.76], p=0.002). There was no difference in 72-hour return rates. Conclusions: Factors beyond abscess size, including location, ultrasound findings, and spontaneous drainage, significantly influence procedural decision-making in the PED; suggesting current size-based guidelines may be incomplete. Notably, black patients were less likely to receive drainage procedures, raising concern for potential disparities in care. Further investigation is needed to determine whether these differences reflect clinical appropriateness or inequities, and to evaluate outcomes of conservative management across diverse patient populations.

Cardiac Bioengineering & Platforms

Associations Between the Gut Microbiome and Hemodynamic Parameters in Neonates with Congenital Heart Disease

Anna Miele, Allison Murphy, Christina J Calamaro, Ann-Marie Brown, Patricia Denning, Scott Gillespie, Hui Huang, Rachael Keiffer, Kolby Sanders-Lewis, Amelia St. John, Jinbing Bai, Michael P. Fundora

Presenting Author: Anna Miele, BA (MD Student)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16403 and 16404
Poster Number: 55

Abstract

Background: The gut microbiome (GM) plays a critical role in metabolism, immune development, and physiologic adaptation in early life. Neonates with congenital heart disease (CHD) are exposed to altered hemodynamics, yet the associations between GM features and hemodynamic status is not well understood. Objectives: To characterize the gut microbiome of neonates with CHD and explore associations between microbiome features and hemodynamic parameters. Methods: A secondary analysis of stool samples from a prospective cohort of 50 neonates with CHD was conducted. Samples were collected preoperatively (T0), postoperatively (T1), and prior to discharge (T2). Microbial DNA was extracted and the 16S rRNA V4 region sequenced. Hemodynamic variables included echocardiographic parameters and vasoactive–inotropic scores (VIS) measured at the time of each stool collection. Alpha- and beta-diversity metrics and relative taxa abundance associated with hemodynamics variables were analyzed using QIIME 2 and Microbiomeanalyst 3.0. Results: Forty-one neonates with CHD were analyzed, 59.3% male, 51.9% White, and 91.4% non-Hispanic. Alpha-diversity was not associated with echocardiographic parameters and VIS scores. Beta-diversity showed marginal differences in normal vs abnormal echocardiographic parameters (Bray-Curtis, F=1.81, p=0.095; Jaccard, F=1.82, p=0.076). Neonates with abnormal ventricular function showed less Enterococcus (p=0.001, padj.=0.006) and more Haemophilus (p=0.006, padj.=0.019). Neonates with abnormal ventricular function and higher VIS scores showed less Streptococcus (p=0.00004, padj.=0.0003) and Haemophilus (p=0.0002, padj.=0.0005). Conclusions: Neonates with CHD seemed to exhibit distinct gut microbiome diversity and specific taxa linked with hemodynamic parameters across the perioperative period. Exploring associations between microbiome features and hemodynamic parameters provides insight into mechanisms linking physiologic instability, microbial dysbiosis, and recovery in this high-risk population.

Oncology & Cellular Therapy

Patterns of Clinical Trial Availability and Enrollment in Adolescents and Young Adults with Hodgkin Lymphoma at Affiliated Pediatric and Adult Cancer Centers

Karpowicz, J; Muniz, JP; Khanna-Farber, A; Hawk, A; Lee, K; Ji, X; Bergsagel, DJ; Fridlyand, D; Scheurer, ME; Allen, P; Koff, J; Blum, K; Arellano, M; and Castellino, SM

Presenting Author: Joshua Muniz, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 27

Abstract

Background: Adolescents and young adults (AYA; ages 15-39) have lower clinical trial enrollment rates compared to younger pediatric patients. Contributing factors may include institutional practices, differences in trial availability between pediatric and adult centers, and social determinants of health. However, limited data exist on how trial availability across treatment centers influences clinical trial enrollment among AYA patients. Methods: We identified a retrospective cohort of 407 AYA patients with newly diagnosed Hodgkin Lymphoma (HL) who presented to our affiliated institutions between 2010-2023. Institutional review board records from Children's Healthcare of Atlanta (CHOA) and Winship Cancer Institute were queried to determine availability of therapeutic frontline HL trials during the study period. Among newly diagnosed AYA patients for whom a frontline clinical trial was open at diagnosis and for which they were trial-eligible (n=100), we examined patient characteristics associated with trial enrollment. Results: At CHOA, 6 frontline trials were open at some point during the study period, depending on cancer subtype and trial open/closure dates; there were no trials for early-stage HL between 2011-2019 and none for advanced-stage HL between 2012-2015. At Winship, 4 frontline trials were open during the study period; there were extended periods lacking any frontline trials (2010-2011, 2013-2018). Among the 407 AYA patients identified, 280 (68.8%) received frontline therapy at either center. Across both institutions, 100 patients (35.7%) were eligible for an open frontline clinical trial. Of these, 25% enrolled in a trial (21.6% at Winship, 28.6% at CHOA). CHOA (vs. Winship) cared for a higher proportion of publicly insured patients (52.6% vs 19.6%, $p<0.01$) and Hispanic patients (22.4% vs. 2.0%, $p=0.02$). Trial enrollment did not differ by age, sex, insurance type, or race/ethnicity. Conclusions: Among AYA patients with HL, clinical trial availability differed by treatment center. However, there were no differences in trial enrollment by treatment center or patient characteristics. These findings highlight the importance of improving consistent and equitable trial availability for AYA patients across pediatric and adult centers. Ongoing work will extend this analysis to other hematologic malignancies in both the frontline and relapsed/refractory settings as part of a cross-system AYA program development.

Gastrointestinal, Hepatic & Renal

Exploring the Relationship Between Imaging-Related Radiation Exposure and the Gut Microbiome in Neonates with Congenital Heart Disease

Murphy, Allison; Miele, Anna; Calamaro, Christina J; Brown, Ann-Marie; Denning, Patricia; Gillespie, Scott; Huang, Hui; Keiffer, Rachael; Sanders-Lewis, Kolby; St. John, Amelia; Bai, Jinbing; and Fundora, Michael P.

Presenting Author: Allison Murphy, BA (MD Student)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16403 and 16404
Poster Number: 66

Abstract

Background: The gut microbiome (GM) is critical in metabolic regulation and development. Neonates with congenital heart disease (CHD) are exposed to ionizing radiation from diagnostics and catheterization. The influence of radiation exposure linked to GM remains largely unknown. Objectives: To characterize radiation exposure in neonates with CHD and explore associations with the GM across the perioperative course.Methods: Secondary analysis of a prospective cohort of 50 neonates with CHD. Stool samples were collected preoperatively (T0), postoperatively (T1), and prior to discharge (T2). Microbial DNA was extracted and 16S rRNA V4 region sequenced. Radiation exposures included portable radiographs (XR) quantified using Exposure Index (EI), computed tomography (CT) quantified by milliampere-seconds (mAs), and cardiac catheterization quantified by dose–area product (cGy·cm²). Associations between radiation exposures and GM were analyzed using QIIME 2 and Microbiomeanalyst 3.0.Results: Between admission and T0, 50 subjects underwent 234 radiographs (mean 5.7; EI 63,378), 9 CTs (7,440 mAs), and 11 catheterizations (401 cGy·cm²). T0 to T1, 506 radiographs (mean 11; EI 57,172), 6 CTs (2,831 mAs), and 6 catheterizations (1,481 cGy·cm²). T1 to T2, 612 radiographs (mean 15; EI 13,894), 3 CTs (1,064 mAs), and 2 catheterizations (476 cGy·cm²). Neonates with more XR exposure showed marginally lower alpha diversity ($p=0.10$). Beta diversity was not associated with exposure. Less XR was associated with higher Streptococcus (padj.<0.001) and Haemophilus (padj. <0.001). Lower EI had more Haemophilus (padj. <0.001) and Streptococcus (padj. <0.001). Those who did not undergo catheterization had higher Streptococcus (padj.$=0.002$), Haemophilus (padj.$=0.002$), and Enterococcus (padj.$=0.022$). Neonates with less XR and EI had more Streptococcus ($p<0.05$) after controlling for confounders.Conclusions: Neonates with CHD experiencing more radiation exposures were associated with differences in the GM. Evaluating the relationship between radiation exposure and GM may provide insight into previously unrecognized contributors to microbial dysbiosis in this high-risk population.

Reproductive, Adolescent & Women's Health

The Effects of Hair-Related Care Products on Adverse Pregnancy Outcomes

Nwaise, Ogugua; Burjak, Chloe; Ogbonna, Julian; Williams, Mi'Angel; and Cordero, José F.

Presenting Author: Ogugua Nwaise, BS (Master's Student)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 45

Abstract

Background: Hair-related personal care products (HPCPs) are widely used and contain chemicals such as phthalates and endocrine-disrupting chemicals (EDCs), which may affect fetal development. This systematic review evaluates the current knowledge on the link between exposure to PCPs and adverse pregnancy outcomes.Methods: Using PRISMA guidelines, we searched for studies that measured exposure to phthalates and other chemicals present among persons using HPCPs in PubMed, Web of Science, and Cochrane Library. Two independent reviewers screened and assessed the studies for bias using the ROBINS-I V2 tool. A meta-analysis of HPCPs and adverse pregnancy outcomes was conducted.Results: A statistically significant increased risk of preterm birth was found among HPCP users (OR: 1.15, 95% CI: 1.02, 1.29) and small for gestational age (OR: 1.34, 95% CI: 1.14, 1.56). No association was found between HPCP exposure and low birth weight.Conclusions: This review underscores the potential risks of HPCP exposure during pregnancy. Limitations of this analysis include the sparse number and diverse study designs, and the unspecified nature of the hair products used. Further research based on cohort studies is needed to examine relationships between hair products and EDCs.

Neurodevelopment & Autism Interventions

An Evaluation of Backward Chaining to Teach a Mand Topography Using a Speech Generating Device (SGD) to a Child on the Autism Spectrum

Ortega, Ryan; Cohen, Jamie; & O'Guinn, K. Nicole

Presenting Author: Kristin OGuinn, PhD (Junior Faculty)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 21

Abstract

Children diagnosed with autism spectrum disorder (ASD) often present with language and communication difficulties. Speech generating devices (SGD) display communication symbols in a multiple-choice format in terms of choosing an icon from an array that matches the individuals' communicative needs. When a user touches or selects a symbol on an SGD, the verbal label of the relevant symbol is activated. SGDs may offer a more technological form of alternative communication, as it allows for more complex language and access to a larger vocabulary. Participants in this study were systematically introduced to an SGD using a backwards chaining procedure to teach verb noun requests. These procedures were effective for two out of the three participants. Data from a current study based on modifications to these procedures will be presented, and Implications for practical application will be discussed.

Cardiac Bioengineering & Platforms

Isolated Polyvalvular Heart Disease Associated with a TAB2 Variant: Expanding the Phenotypic Spectrum of TAB2-Related Cardiovascular Disease

Patel, Paula; Lanza, Julia; Demo, Erin; Sallee, Denver; Iannucci, Glen

Presenting Author: Paula Patel, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 4

Abstract

BackgroundPolyvalvular heart disease (PVD) has historically been associated with syndromic conditions such as Trisomy 13, Trisomy 18, and connective tissue disorders. Emerging evidence suggests that rare, single-gene variants—including FLNA and TAB2—can underlie isolated or familial valvular disease. TAB2 mutations have primarily been reported in syndromic congenital heart disease (CHD) with extracardiac anomalies. However, nonsyndromic presentations remain under recognized.CaseWe report a 20-year-old nonsyndromic female with a history of PVD identified at age 6 following evaluation for palpitations. Early echocardiography revealed mitral valve prolapse (MVP) without regurgitation and tricuspid valve prolapse with mild regurgitation. By age 13, echocardiography demonstrated redundant dysplastic atrioventricular valves with mild regurgitation across all valves. Physical exam revealed hypermobility (Beighton score 9) without other cutaneous or skeletal features of a connective tissue disorder. Genetic testing was negative for FLNA variants.A three-generation family history revealed MVP and progressive valvular disease in the patient's mother and maternal aunt. The aunt, who experienced heart failure and required implantable cardioverter-defibrillator (ICD) placement and ablation for ventricular arrhythmias, was found to carry a heterozygous nonsense variant in TAB2 (c.430C>T; Q135X). This variant is absent in large population databases and is classified as likely pathogenic. Cascade testing identified the variant in the proband and mother, confirming segregation with disease. The maternal grandmother lacked the variant and was asymptomatic. ResultsThe TAB2 variant segregated with disease in the family, with affected individuals demonstrating progressive valvular dysfunction, including MVP, tricuspid valve disease, arrhythmias, and cardiomyopathy. Notably, there were no extracardiac anomalies, distinguishing this presentation from classic syndromic TAB2-related disease. Prior reports have highlighted the presence of developmental delays and this was not present in this family, with all affected members completing college degrees.ConclusionThis case expands the phenotypic spectrum of TAB2-associated cardiovascular disease to include nonsyndromic, isolated polyvalvular heart disease with connective tissue features. It highlights the value of cascade genetic testing and comprehensive family history in evaluating PVD of uncertain etiology. Clinicians should consider TAB2 as a candidate gene in familial valvular disease—even in the absence of syndromic features—to guide early diagnosis, surveillance, and risk stratification.

Cardiac Bioengineering & Platforms

Human iPSC-Derived Cardiac Models Enable Predictive Assessment of Tyrosine Kinase Inhibitor–Induced Cardiotoxicity

Patel, Gayatri; Zhang, Wenhao; Reid, Olivia; Li, Stephanie; Ling, Zhi; Park, Sung Jin; Du, Yuhong; Mandawat, Anant; Jia, Shu; Maxwell, Joshua and Xu, Chunhui

Presenting Author: Gayatri Patel, MS (PhD Student)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16403 and 16404
Poster Number: 56

Abstract

BACKGROUND: Chronic myelogenous leukemia (CML) is a rare hematologic malignancy that presents unique clinical challenges due to the need for long-term therapy during critical stages of growth and cardiac development. Tyrosine kinase inhibitors (TKIs) such as nilotinib, commonly prescribed in children, while ponatinib is recommended cautiously, have significantly improved survival. However, nilotinib shows moderate cardiovascular risk, whereas ponatinib exhibits more severe toxicity, with 878 and 951 cardiovascular adverse event reports, respectively, recorded in the FDA Adverse Event Reporting System for 2025. Understanding the mechanisms of TKI-induced cardiotoxicity using relevant three-dimensional cardiac models is essential to improve long-term quality of life in pediatric CML patients. OBJECTIVE: To evaluate the dose-dependent cardiotoxic effects of nilotinib and ponatinib in 2D and 3D human induced pluripotent stem cell–derived cardiomyocytes (hiPSC-CMs). METHODS: hiPSCs were differentiated into early-stage cardiomyocytes and assessed for differentiation efficiency and cellular purity. Both 2D and 3D hiPSC-CM models were treated with increasing concentrations of nilotinib and ponatinib for defined exposure periods. Cardiotoxicity was assessed through measurements of cell viability, mitochondrial stress, ATP content, contractile function, including contractility and relaxation velocity. RESULTS: Nilotinib and ponatinib reduced cell viability in a dose-dependent manner in 2D differentiation cultures containing >77% cardiomyocytes. Ponatinib treatment resulted in a dose-dependent increase in mitochondrial reactive oxygen species. In addition, high concentrations of nilotinib caused a significant loss of mitochondrial membrane potential, leading to cardiomyocyte depletion, as evidenced by reduced expression of cardiac markers NKX2.5 and a-actinin. Consistent with these observations, 3D cardiac spheroids exhibited a marked reduction in ATP content over a five-day exposure period to both TKIs, accompanied by pronounced morphological alterations. Functional assessment of 3D cardiac spheroids revealed drug-specific cardiotoxic profiles: nilotinib exposure impaired contraction and relaxation velocities within 24 hours, while ponatinib significantly disrupted contractility after 72 hours. CONCLUSION: Although TKIs such as nilotinib and ponatinib have revolutionized CML treatment, their associated cardiotoxicity poses a significant barrier to long-term patient health and quality of life. This study highlights the utility of hiPSC-CM platforms for predictive cardiotoxicity screening with the future aim of exploring potential cardioprotectants to improve the safety profile of CML treatments.

Pulmonary, Respiratory Support & Cystic Fibrosis

Signaling Modulation in M. tuberculosis–Infected Human Macrophages: A Comparison of Standard and Airway-Conditioned Models

Pickering, Nola; Leese-Thompson, Collin; Tirouvanziam, Rabindra

Presenting Author: Nola Pickering, BS (Undergraduate student)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 32

Abstract

BACKGROUND: Mycobacterium tuberculosis (Mtb) typically enters via the respiratory tract and reaches the lung, where it is engulfed by alveolar macrophages (AMs). Infection mediates changes in immune signaling and phenotypic expression in AMs. Of interest are the PD-1/PD-L1 and CD200/CD200R pathways, which have been shown to inhibit M1 activation and induce M2 polarization in tumor-associated macrophages, though study of their role in Mtb infection remains limited and contentious. Many studies employ human blood monocyte-derived macrophages (BMDMs) in infection assays, which do not account for phenotypic differences present in airway macrophages. However, an airway-like phenotype can be achieved via transmigration through an epithelium, producing airway-resident macrophages (ArMas). This study examines both phenotypic changes induced by Mtb infection and differences between classically produced, non-transmigrated, BMDMs and ArMas.METHODS: Human monocytes were isolated from whole blood. To achieve airway conditioning, monocytes were transmigrated across an air–liquid interface human small airway epithelium cultured on Alvetex scaffolds toward media containing the monocyte chemoattractant CCL2. Blood monocytes and transmigrated monocytes were differentiated into BMDMs and ArMas, respectively, using M-CSF, and subsequently infected with CFSE-stained Mtb H37Ra at MOI 3. Infection rate and phenotypic expression were characterized by flow cytometry, and bacterial burden was assessed using a colony forming unit assay.RESULTS: BMDMs displayed suppressed expression across signaling pathways following Mtb infection. In contrast, ArMas displayed suppression in M1-typical pathways and increased expression in M2-typical pathways, including elevated PD-1/PD-L1 and CD200/CD200R expression. ArMas also exhibited a higher bacterial burden relative to BMDMs.CONCLUSIONS: Few studies investigating macrophage responses to Mtb infection account for phenotypic differences resulting from airway conditioning. Here, airway conditioning resulted in an attenuated M2-consistent phenotype and increased bacterial burden during Mtb infection, consistent with typical properties of tissue-resident macrophages which may prioritize immune tolerance over microbicidal clearance. This contrasted with the global suppression of immune receptor expression observed in Mtb-infected BMDMs. Our findings emphasize the utility of simulated airway conditioning to enhance physiologic relevance in in vitro human macrophage models of Mtb infection, and support a potential attenuating role for PD-1/PD-L1 and CD200/CD200R signaling during Mtb infection in airway-conditioned macrophages.

Public Health, Equity & Care Delivery

Language Justice for Non-English or Spanish Speaking Families on the Hospital Medicine Service

Potter, Andrew; Hames, Nicole; and Rule, Amy

Presenting Author: Andrew Potter, DO (Medical Fellow)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16503 and 16504
Poster Number: 97

Abstract

Background: Georgia is home to roughly 850,000 immigrants, including many refugee families. Immigrants face unique challenges in health care every day, such as barriers to transportation, access to medicine, and language barriers in utilizing health services. Hospitalized immigrant patients and their families face these barriers while dealing with the additional stressor of an acutely ill child. This quality improvement project is aimed towards language justice for families that speak languages other than English or Spanish. While families admitted to CHOA are asked their preferred learning methods and are screened for literacy, it is unclear how many receive education consistent with those needs. Methods: We conducted preliminary data collection utilizing chart review from June 2025 – November 2025 for all clinical admissions to Children's Healthcare of Atlanta – the 3 inpatient campuses. The top 5 most common languages spoken in hospitalized CHOA patients and families includes Amharic, Arabic, Vietnamese, French, and Swahili (from July 2025 – November 2025). Using EPIC Slicer-Dicer, we parsed out encounters with a preferred language for Amharic or Arabic as an initial pilot needs assessment. Families preferred learning method, literacy assessments, and whether they received discharge instructions in their preferred language were compiled and analyzed used descriptive statistics. Results: Of the 62 unique families that were admitted to CHOA who spoke either Amharic or Arabic, 55% chose "reading" as a preferred learning method. Along with that, 30% of these 62 families indicated that they "sometimes, often, or always" require assistance reading instructions. Overall, of the 62 unique families that spoke Amharic or Arabic, 0 received discharge instructions in their own preferred language. Conclusions: It is imperative that we develop culturally responsive and preferred-language congruent child health education resources in multiple languages. As part of our language justice initiative, this needs assessment further outlines the desire for immigrant families to have reading materials in their preferred language, and next steps for this pilot project involve utilizing local Amharic and Arabic interpreters to translate common pediatric diagnosis discharge instructions with cultural context in mind and implementing their use on the hospital medicine service at CHOA.

Neurodevelopment & Autism Interventions

Evaluating Factors that Predict the Feasibility, Acceptability, and Appropriateness of Two School-based CBT Programs for Autistic Students

Pu, Michelle; Boles, Richard; Islam, Nailah; Menon, Nina, Myers, Sarah; Kolios, Alexander; Klinger, Laura; Reaven, Judy; Pickard, Katherine

Presenting Author: Michelle Pu, BS (Predoctoral Fellow)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16501 and 16502
Poster Number: 89

Abstract

Background:Autistic youth experience high rates of anxiety. Although cognitive behavioral therapy (CBT) reduces anxiety in autistic individuals, access is often limited. School-based CBT is a promising avenue to reduce barriers to access, yet schools face implementation challenges. We evaluated implementation outcome ratings of two school-based CBT programs, Facing Your Fears in Schools (FYF-S) and Zones of Regulation (ZOR). We hypothesized that FYF-S would demonstrate higher implementation ratings than ZOR and that provider- and school-level factors would predict implementation outcomes. Methods: Interdisciplinary School Providers (ISPs) in North Carolina and Colorado participated in an ongoing comparative effectiveness trial of ZOR and FYF-S for autistic students with anxiety. ISPs completed baseline questionnaires (n=175) assessing demographics, prior CBT training and use, stress and burnout, attitudes towards evidence-based practices, and school leadership and climate. Following intervention delivery, ISPs (n=100) completed an Implementation Survey measuring Acceptability, Appropriateness, and Feasibility. Independent-samples t-tests examined intervention differences, with ANCOVAs when appropriate. Multivariable linear regressions identified provider- and school-level predictors. Results:FYF-S Acceptability was rated higher than ZOR ($t(98)=2.32$, $p=.022$, $d=.47$) and remained significant after controlling for location by state $F(1, 97)=4.60$, $p=0.034$). No differences emerged for Appropriateness or Feasibility. Implementation Climate was marginally associated with Appropriateness, accounting for location ($R2=.044$, $F(2,95)=3.25$, $p=.057$; β =.191). Mental health professional status initially predicted Feasibility ($R2=.069$, $F(1,97)=7.217$, $p=.008$; β =.263) but was nonsignificant with location ($p=.071$; β =.189), as there were significantly more mental health providers in CO. Providers in CO had higher ratings of Feasibility ($R2=.107$, $F(1,96)=4.09$, $p=.046$; β =-.209), greater CBT exposure ($t(173)=6.12$, $p<.001$, $d=1.07$) and marginally increased experience in formal CBT training ($t(166)=1.98$, $p=.050$, $d=1.17$) in comparison to providers in NC. Conclusions:FYF-S was perceived as more acceptable than ZOR. The role of school-level and provider factors indicates that tailored implementation strategies are needed to support school-based CBT for autistic students.

Pulmonary, Respiratory Support & Cystic Fibrosis

Assessment of Handgrip Strength as a Potential Functional Marker of Musculoskeletal Health in Pediatric Cystic Fibrosis

Purohit, Neha; Carrick, Erin; Joshi, Rajeshree; Crandall, Reva; Wimmer, Nicole; Harris, Ryan; Purohit, Sharad; McKie, Kathleen; and Adair, Dionne

Presenting Author: Neha Purohit, BS (Undergraduate student)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16501 and 16502
Poster Number: 85

Abstract

Background: Children and adolescents with cystic fibrosis (CF) are at risk for impaired muscle strength, altered body composition, and reduced bone mineral density (BMD). Conventional clinical measures do not adequately reflect functional muscle deficits. Clarifying relationships among lean mass, physical activity, bone health, and muscle strength may improve identification of musculoskeletal risk in pediatric CF. This study assesses muscular strength and lean body mass in pediatric CF patients at our center and examines correlation between these physiological markers and reported levels of physical activity and lung function.Methods: This single-center, cross-sectional, IRB-approved study enrolled 40 children and adolescents with CF (ages 8–21 years). All participants were receiving CFTR modulator therapy and had preserved pulmonary function. Lean mass (LM) was assessed using bioelectrical impedance analysis, and muscle strength (HGS) was measured via handgrip dynamometry. Measures were normalized to appropriate age- and sex-based reference values and expressed as Z-scores. Physical activity (PA) was assessed using validated age-specific questionnaires with the cohort divided into younger (PAQ-C; ages 8–14 years) and older (IPAQ; ages 15–21 years) groups. BMD Z-scores were obtained from clinical records in a subset of participants (n=10). Pulmonary function was assessed using percent predicted FEV1 ($ppFEV_1$). Associations were evaluated using Pearson correlation analyses and Analysis of Variance (ANOVA).Results: PA levels were significantly associated with HGS ($F(3,32)=6.625$, $p=0.001$) and LM index ($F(3,32)=5.006$, $p=0.006$). LM moderately correlated with HGS in younger participants ($r=0.431$, $p=0.035$), but not in older participants. Although HGS trended toward a positive association with BMD, this correlation was not statistically significant. $ppFEV_1$ did not differ significantly across HGS groups, although a stepwise numerical increase in median and mean $ppFEV_1$ was observed from marked weakness to normal strength categories.Conclusions: Handgrip strength demonstrates positive relationships with lean mass, physical activity, and bone health in pediatric CF. Handgrip dynamometry is a practical, noninvasive functional measure that may complement traditional anthropometric assessments, particularly when more resource-intensive testing is not feasible. Incorporating functional strength assessment into routine CF care may enhance early identification of musculoskeletal risk and help identify patients who could benefit from targeted exercise interventions or physical therapy.

Data Science, Methods & Biomarkers

Ventilator Data Downloads in Children and Young Adults with Central Hypoventilation Syndromes

Raj, Niketa; Harford, Kelli; Leu, Roberta; Kasi, Ajay; and Shah, Amit

Presenting Author: Niketa Raj, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 53

Abstract

Background: Congenital central hypoventilation syndrome (CCHS) and rapid-onset obesity with hypothalamic dysfunction, hypoventilation, and autonomic dysregulation (ROHHAD) are rare disorders affecting respiratory control that require lifelong ventilatory support. Due to abnormal ventilatory responses, patients do not increase their minute ventilation despite hypoxemia and hypoventilation. Both conditions carry significant morbidity and mortality risk if hypoventilation is inadequately managed. Ventilator data downloads provide valuable information on compliance and respiratory parameters, yet there is limited standardized evaluation of this data in children. We hypothesize that evaluation of ventilator compliance in ventilator data downloads impacts clinical management in children and young adults with CCHS and ROHHAD. The study aimed to evaluate ventilator compliance and respiratory parameters obtained from ventilator data downloads in patients with CCHS and ROHHAD.Methods: This retrospective study evaluated children and young adults aged 0-21 years with CCHS and ROHHAD who received care at Children's Healthcare of Atlanta between January 2024 and December 2025. Eligible subjects had at least one ventilator data download during the study period. Data collected included ventilator compliance metrics (percent days used, average hours used per day, minimum and maximum usage), respiratory parameters (tidal volume, minute ventilation, respiratory rate, pressures, and leak volume), and clinical management changes based on download results (such as referral to sleep psychologist to promote ventilator compliance, hospitalization, nighttime home healthcare nursing, and changes in mask interface or ventilator settings). Descriptive statistics will summarize compliance patterns and respiratory parameters. Comparison groups will include noninvasive positive pressure ventilation versus ventilation via tracheostomy, age groups, and duration since ventilation initiation.Results: We identified 25 subjects (20 with CCHS and 5 with ROHHAD) who had ventilator data downloads during the study period. Data collection is ongoing.Conclusions: Understanding patterns of ventilator usage and respiratory parameters may identify patients at risk for inadequate ventilator use, thereby promoting early clinical interventions such as counseling, mask assessment, ventilator setting adjustments, and mental health screening. This study will contribute to evaluation of ventilator data download interpretation across different ventilator models and has the potential for incorporation into routine clinical care.

Reproductive, Adolescent & Women's Health

To Study the Prevalence and Risk Factors for Prehypertension and Hypertension among Adolescents (18–19 years) at Entry Level of Professional Course

Reshi,Amrutha ; Y. V, Abhijith; N. Chandra, Sharath; A. C, Ramesh and D. M; Narayanaswamy

Presenting Author: Amrutha Reshi, MBBS

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 46

Abstract

Aim and Objective: To study the prevalence and risk factors of prehypertension and hypertension among adolescents (18–19 years) at the entry level of professional course. Materials and Methods: After institutional ethics committee approval and informed consent from participants, the data were collected and entered into the Excel sheet. Appropriate statistical analysis was applied. The final result was statistically interpreted.Results: A total of 273 student participants were included in the study. There were 104 males and 169 females. One hundred and seven participants were 18 years old and 168 were 19 years old. Male: female ratio is 1:1.6. Prehypertension was seen in 91 (33.3%) and hypertension was observed in 34 (12.57%). Seven (20.6%) cases had a family history of hypertension. Twenty-two (8.1%) had addictive habits. Inadequate physical activity was seen in 32 (94.1%) of cases who had hypertension. Poor sleep quality was seen in hypertensives (17; 50%). In our study also, there was no statistically significant association between gender and systolic blood pressure and diastolic blood pressure ($P > 0.05$). Thereis a statistical significance association between the gender and body mass index (BMI) but not between BMI and hypertension status. There was statistically significant association was there between prehypertension and hypertension with habits. Conclusion: Change in habits and dietary modification will have impact on the development of prehypertension and or hypertension among adolescents. More emphasis must be given to screen adolescents at college entry. Adolescents are best target age group for primordial prevention.Keywords: Adolescents, cardiovascular diseases, exercise, hypertension, lifestyle modification.

Gastrointestinal, Hepatic & Renal

Acute Kidney Injury and Demographic Differences in Pediatric Acute Gastroenteritis

Rose, Daniel I.R.; Shin, H. Stella

Presenting Author: Daniel Rose, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 12

Abstract

Acute gastroenteritis (AGE) is a common pediatric illness that can cause dehydration and acute kidney injury (AKI), both associated with increased health care utilization. Emergency department (ED) risk stratification for AKI varies across institutions and often relies on subjective assessments. Limited data differentiate clinical indicators that increase risk for AKI in AGE. We aimed to describe characteristics of pediatric patients hospitalized with acute gastroenteritis and AKI to inform future biomarker-based risk stratification studies. We conducted a retrospective cohort study of pediatric patients aged 0-18 years presenting to the Children's Hospital of Atlanta (Children's) ED between 2018-2024. Patients admitted to the pediatric hospital medicine service with a diagnosis of acute gastroenteritis were included. Encounters at Children's Urgent Care locations and patients with sepsis, shock, or chronic illness were excluded based on ICD-9 and 10 codes in past medical history. We examined demographics, ED disposition, length of stay, insurance type, fluid resuscitation, and incidence of AKI defined by KDIGO criteria. Among 108,637 eligible ED visits, 5.5% were admitted for AGE, and 8.3% of admitted patients developed AKI. The mean age was 4.37 years (SD 4.52). Among patients with AKI, racial distribution was Black/African American (B/AA) 34.60%, White/Caucasian (WC) 32.70%, and Multiracial Hispanic (MH) 15.56% compared to the institution's overall ED population with those patients identifying as B/AA 47.51%, WC 19.29%, and MH 19.28%. Patients admitted for AGE identified as WC 38.64%, B/AA 32.19%, and MH 15.06%. Biological sex distribution was 52.33% male (M) and 47.68% female (F) among patients presenting to the ED, and 50.84% M and 46.93% F among those who were admitted. Insurance types were public 68.88% and private 26.20%. Additional multivariable analyses are ongoing. A subset of children hospitalized for AGE developed AKI, highlighting the need for improved early risk stratification. Differences between the racial distribution of children presenting to the ED and those admitted warrant further investigation to assess the impact of social factors on AGE management. Objective biomarkers may improve identification of children at the highest risk and reduce variability in admission decisions.

Neurodevelopment & Autism Interventions

Features of Developing Saccade Control are Differentially Related to Maturation in Cortical White Matter Tracts

Saad, Moura; Shultz, Sarah; and Ford, Aiden

Presenting Author: Moura Saad, BA (Predoctoral Fellow)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16501 and 16502
Poster Number: 91

Abstract

Background:Saccade control (SC) development is a significant process in early infancy, permitting increased visual search, attentional deployment, and ultimately, visual learning. By 2-3 months, infants track objects and require fewer saccades to reach targets. While literature has characterized large-scale changes in SC, there is limited understanding of how cortical maturation is related to SC in early infancy. This study leverages longitudinal measures of infant white matter development and volitional SC to identify white matter tracts associated with different features of SC. Methods:Longitudinal diffusion-weighted imaging and calibration-based eye-tracking data were collected from the same 73 neurotypically developing infants, at up to 3 and 5 timepoints, respectively. From the eye-tracking data, three measures of SC were extracted: saccade amplitude, percent of targets reached, and number of saccades to targets. Seven white matter tracts associated with visual processing and/or motor control were delineated using probabilistic tractography: Anterior Thalamic Radiation (ATR), Body, Genu, and Splenium of the Corpus Callosum (CCb, CCg, and CCs), Inferior Fronto-occipital Fasciculus (IFOF), and motor and somatosensory subdivisions of corticofugal tracts (M1 and S1). Developmental trajectories of white matter maturation (indexed via fractional anisotropy) and change in SC measures were constructed using Functional Principal Component Analysis, and brain-behavior relationships between trajectories were modeled using Functional Linear Regression (Yao and Wang 2005a,b). Results:Exploratory FLR models show that maturation of the CCg and CCs were predictive of saccade amplitude from 0-6 months ($p<=0.1$) and maturation of S1 was predictive of percent of targets reached ($p<=0.1$). Time-varying R2 functions showed that the CC tracts explained the most variance in saccade amplitude from 2-3 months and S1 explained the most variance in percent of targets reached from 4-6 months. Conclusion:Different white matter tracts are associated with different features of SC. Saccade amplitude, showing the least developmental change, was associated with two areas of the corpus callosum (CCg and CCs). Percent targets reached, showing a rapid increase from 0-6 months, was associated with S1. Future analyses will use a penalty-based metric to examine saccade efficiency in neurotypical infants and infants later diagnosed with autism, highlighting potential between-group oculomotor differences.

Oncology & Cellular Therapy

Utilizing PTK7-directed CAR gamma delta T cells to target T-cell acute lymphoblastic leukemia

Schiaffino Bustamante, Austre; Fedanov, Andrew; Fanelli, Brandon; Skinner, Katie; Doering, Christopher; Spencer, Trent; and Raikar, Sunil

Presenting Author: Austre Schiaffino Bustamante, BS (PhD Student)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 28

Abstract

BACKGROUND: The clinical success of chimeric antigen receptor (CAR) based cellular immunotherapies for T-cell acute lymphoblastic leukemia (T-ALL) has been limited due to the lack of T-ALL-specific surface antigens. **γδ** T cells are a promising alternative CAR platform due to their innate anti-leukemic properties and MHC-independent mode of action; however, limited bone marrow trafficking and in vivo persistence remain significant challenges. We identified protein tyrosine kinase 7 (PTK7) as a potential target in T-ALL and previously demonstrated that **γδ** T cells expanded in the presence of transforming growth factor beta (TGF**β**) exhibit enhanced persistence and increased bone marrow trafficking. Here, we describe a novel cellular immunotherapy strategy utilizing TGF**β**-expanded **γδ** T cells engineered to express a PTK7-directed CAR to improve anti-T-ALL activity. METHODS: PTK7 was identified through RNA sequencing by comparing the transcriptional profiles of T-ALL samples and **γδ** T cells. PTK7 protein expression was validated by Western blot and flow cytometry. **γδ** T cells were expanded in the presence of TGF**β** and transfected to express a PTK7 CAR. CAR expression was quantified by flow cytometry, and cytotoxic activity of TGF**β**-expanded PTK7 CAR **γδ** T cells against T-ALL cell lines was assessed using a flow cytometry-based cytotoxicity assay. RESULTS: Differential gene expression analysis confirmed upregulation of PTK7 in T-ALL samples, with low expression observed in **γδ** T cells. Western blot and flow cytometry demonstrated PTK7 expression at both total protein and cell surface levels in T-ALL cell lines. CAR expression was higher in TGF**β** expanded **γδ** T cells compared to non–TGF**β** expanded controls (92.8% CAR+ vs 46.2% CAR+, respectively), and this was associated with enhanced cytotoxic activity against T-ALL cell lines (%cytotoxicity 85.9% vs 57.3%, respectively)CONCLUSIONS: PTK7 has previously been studied in our group as a promising immunotherapy target for pediatric solid tumors. Here, we demonstrate that PTK7 is highly expressed in T-ALL and can be effectively targeted using TGF**β**-expanded PTK7 CAR **γδ** T cells. These findings support the feasibility of utilizing TGF**β**-expanded **γδ** T cells as an alternative CAR product and highlight PTK7 as a potential target for the development of novel cellular immunotherapies for T-ALL.

Gastrointestinal, Hepatic & Renal

Depression, Anxiety and Stress Among Caregivers of Children with Chronic Kidney Disease and its Impact on Kidney Failure Treatment Decisions: A Cross-Sectional Survey Study

Schnaith, Abigail MD; Urbanski, Megan PhD MSW; Reed, Bonney PhD; Huang, Hui, MS; Westbrook, Adrianna MPH; Garro, Rouba, MD; Kennedy, Sabina MD; Greenbaum, Larry A MD, PhD; and Wang, Chia-shi MD MSc.

Presenting Author: Abigail Schnaith, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 13

Abstract

Background: Caregivers of children with chronic kidney disease (CKD) are asked to make important kidney failure treatment decisions, including type of dialysis (hemodialysis [HD] or peritoneal dialysis [PD]) and type of kidney transplant (living donor vs. deceased donor). Dialysis choice may affect quality of life; living donor kidney transplants have superior outcomes, including longer graft survival compared to deceased donor kidney transplants. Family stress and poor caregiver mental health may be barriers to living donation in adult kidney failure patients. Little is known about the prevalence of depression, anxiety and stress in caregivers of pediatric patients with chronic kidney disease (CKD) and its impact on kidney failure treatment decisions.Methods: We administered a survey to caregivers (legal guardians) of children <18 years with CKD III-V inclusive of dialysis (peritoneal dialysis, PD; hemodialysis, HD) at outpatient clinics to assess presence of depression, anxiety and stress among caregivers using the Depression Anxiety & Stress Scale [(DASS-21): depression ≥ 10, anxiety ≥ 8 , stress ≥15]. We explored the association between caregiver mental health with reported kidney failure treatment preference (e.g.,kidney donation type, donation timing, dialysis modality). Enrollment began 8/1/2025 and is ongoing. We will describe the distribution of parental demographic and socioeconomic and patient clinical factors by parental mental health status. Univariate logistic regression will be performed to examine associations between caregiver depression, anxiety and stress on kidney failure treatment preference. Significance level set at alpha = 0.05. Results: Currently, 41/44 (93%) caregivers approached consented to the study: 11/12 HD, 12/13 PD, 18/19 CKD, with 37 (90%) completing the survey (30 mothers, 7 fathers). Median age of child at time of survey was 8.7y (IQR 2.7, 13.1) with median duration of CKD of 3.1 years (IQR 2.0, 6.8). Based on the DASS-21 scores, 14 (37.8%) caregivers reported depression, 18 (48.6%) reported anxiety, and 19 (51.4%) reported stress. Conclusions: Stress, anxiety and depression are common in the caregivers of children with CKD. At the conclusion of our study, we will determine if there is an association between caregiver mental health and kidney failure treatment decisions.

Reproductive, Adolescent & Women's Health

Performance of the Adnexal Torsion Prediction Score in the Pediatric Population

Scott, A. Hunter, MD; Landever, Olivia; Smith, Allen D.; Sokkary, Nancy, MD: Johnson, Laura; and Alemayehu, Hanna, MD

Presenting Author: Andrew Scott, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 47

Abstract

IntroductionAdnexal torsion (AT) is a common gynecologic emergency, accounting for up to 3% of gynecologic emergencies in children and requires prompt diagnosis and treatment to prevent potentially life altering long term consequences. With large variability in presentation, efforts have been made to provide objective data to stratify the risk of AT. leading to the development of the Adnexal Torsion Prediction Score (ATPS) that accounts for emesis, ovarian volume, and ovarian volume ratio demonstrating that a score of 0-1, 2-3, and ≥4 indicate risks of torsion of <3%, 3-1-%, and >25%, respectively (2). We compared the ATPS to findings in surgery to further validate the use of the ATPS.MethodsA retrospective chart review was performed of suspected AT in pediatric patients >/=18 years from January 1, 2014 to December 31, 2023. Inclusion criteria were female patients who presented with clinical suspicion for AT, evaluated by ultrasound with ovarian volumes reported on radiology reports. Patients without volume data were excluded. Disposition and operative details were recorded including discharge from the emergency department and diagnosis of AT on surgical reports. Patients who did not undergo surgery were assumed not to have AT. Diagnostic performance was assessed using logistic regression and receiver operating characteristic (ROC) curves. ResultsOf the 287 patients evaluated, 163 had data available. The odds ratio (OR) of AT for the group scoring 2-3 on ATPS was 2.83 (95% CI=1.14-7.06), and the OR of AT in the group scoring ≥4 was 31.8 (95% CI=8.15-124.21). The area under the ROC curve was 0.80. Classification was further improved after adjusting for doppler flow and radiologist interpretation (ROC=0.88).ConclusionThe ATPS is a useful diagnostic tool with excellent performance and can be used to diagnose AT in patients presenting with lower abdominal and pelvic pain. Further investigation is warranted to increase the ability of radiologists to provide the information required to provide the data for calculation of the ATPS. Schwartz, Beth I., et al. "Creation of a Composite Score to Predict Adnexal Torsion in Children and Adolescents." Journal of Pediatric & Adolescent Gynecology, vol. 31, no. 2, 2018, pp. 132–37, https://doi.org/10.1016/j.jpag.2017.08.007.

Reproductive, Adolescent & Women's Health

Ultrasound Impact on Diagnosis of Ovarian Torsion

Scott, A. Hunter, MD; Botchway, Maame T., MBChB; Smith, Allen D.; Landever, Olivia; Raikot, Swathi; Sokkary, Nancy, MD; Johnson, Laura, PhD; and Alemayhu, Hanna, MD

Presenting Author: Andrew Scott, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 48

Abstract

IntroductionAdnexal torsion (AT) accounts for up to 3% of pediatric gynecologic emergencies (1). Transabdominal ultrasound (TAUS) has been the primary tool in evaluation of AT, and with a reported sensitivity and specificity of 90% and 68.7%, respectively, in the pediatric population (2). Difficulties in diagnosing AT on TAUS include inability to visualize both ovaries, and ovarian enlargement due to the presence of cysts or other masses. We aim to describe the diagnostic performance of TAUS in our pediatric population.MethodsA retrospective cohort study was performed of suspected AT in girls 18 years of age and who evaluated with TAUS from January 1, 2014 to December 31, 2023 . Disposition and operative details were also recorded including discharge from the emergency department and diagnosis of AT on diagnostic laparoscopy or laparotomy. The diagnostic performance of TAUS was calculated based on surgical reports. Patients who did not undergo surgery were assumed not to have had AT. Sensitivity, specificity, negative predictive value (NPV), and positive predictive value (PPV) were calculated overall and by menarchal status.ResultsOf the 228 patients, 174 patients were ultimately diagnosed with AT via diagnostic laparoscopy or laparotomy. 54 patients who underwent surgery with the pre-operative diagnosis of AT did not have AT on surgical exploration. The sensitivity, specificity, PPV, and NPV of TAUS for diagnosis of AT for all patients were 94%, 65%, 90%, and 76%, respectively. Specificity was lower among postmenarchal patients (0.60, 95% CI .45-.74) compared to premenarchal patients (0.83, 95% CI 0.54-1.0).ConclusionWhile sensitivity remains high for pediatric patients, the specificity diagnostic imaging can be improved to reduce the number of patients who undergo surgical exploration for AT. Further investigation into low-cost imaging modalities is warranted to improve diagnostic capabilities for AT.Breech LL, Hillard PJ. Adnexal torsion in pediatric and adolescent girls. Current Opinion in Obstetrics and Gynecology. 2005; 17 (5): 483-489. doi: 10.1097/01.gco.0000179666.39548.78.Jourjon, Rebecca, et al. “Analysis of Clinical and Ultrasound Determinants of Adnexal Torsion in Children and Adolescents.” Journal of Pediatric & Adolescent Gynecology, vol. 30, no. 5, 2017, pp. 582–90, https://doi.org/10.1016/j.jpag.2017.03.142.

Cardiac Bioengineering & Platforms

Identifying gaps in prenatal counseling of single ventricle disease

Shen, Brenda MD; Coolidge, Nicole CPNP; Corder, Shelby CPNP; Ro, Sanghee MD

Presenting Author: Brenda Shen, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 5

Abstract

Introduction/Background: Prenatal detection and management of single ventricle lesions is becoming more common with advancing technology. A broad range of topics are covered during these counseling sessions, ranging from cardiac anatomy, surgical intervention, and long-term outcomes. There is limited literature exploring parental experiences and perceptions of prenatal counseling.Hypothesis: This study aims to identify gaps in prenatal counseling for families receiving a fetal diagnosis of a single ventricle lesion. We hypothesize that there will be identifiable gaps in prenatal counseling based on parental surveys.Methods/Approach: An online survey was distributed via email to families who have a living child with single ventricle disease in the interstage period. Demographic data, assessments of counseling effectiveness, and open-ended prompts captured qualitative responses regarding emotional experiences and perceived gaps during their prior prenatal counseling.Results/Data: Among early respondents (n = 10), almost all parents (9, 90%) reported an adequate to excellent understanding of the diagnosis following prenatal counseling and a majority (60%) found the initial counseling session to be "very helpful". Visual aids and access to direct contact information for the care team were each cited as helpful by at least 40% of respondents. When asked about what additional resources respondents would have been helpful, parents specifically commented on a desired connection with mentor families (20%), more discussion of positive outcomes (10%), and requested clearer explanations of day-to-day expectations and long-term planning ((10%). Common emotional responses described in 90% of parents included feeling sad, awful, devastated, overwhelmed, and terrified. Communication was generally described as clear, compassionate, and emotionally sensitive, though a few respondents expressed a desire for more candid and detailed information regarding long-term expectations.Conclusions: Parental feedback highlights that emotional support, visual aids, and care team accessibility were highly valued. However, greater specificity, long-term planning, and honesty about outcomes remain critical areas of need. These findings support the development of more tailored, empathetic, and informative counseling strategies to better support families during a challenging and emotionally complex time.

Pulmonary, Respiratory Support & Cystic Fibrosis

Characterization of Ventilator-Associated Tracheitis and Ventilator-Associated Pneumonia in Mechanically Ventilated Infants in a Level IV NICU

Shin, Grace; Townsend, Janae; Floyd, Chase; Solis Solis, Jaquelin; Piazza, Anthony; and Dariya, Vedanta

Presenting Author: Grace Shin, BA, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 33

Abstract

Background: Mechanical ventilation (MV) is a common, life-saving intervention for critically ill neonates. Prolonged MV increases the risk of ventilator-associated pneumonia (VAP) and ventilator-associated tracheitis (VAT). VAP has been shown to increase the length of hospitalization, hospital cost, neonatal morbidity and mortality. Antibiotics used to treat repeat encounters of VAT/VAP increase the risk of development of multi-drug resistant organisms. With the lack of a current NICU-specific definition for VAP, we postulate an under-reporting of VAT/VAP diagnoses within the electronic medical record and significant variability in management practices. We hypothesize that accurate characterization of VAT/VAP and the adoption of a standardized diagnostic and therapeutic pathway in our NICU will potentially improve antibiotic stewardship and resource utilization. Methods: A retrospective chart review will be conducted on NICU patients who were MV via endotracheal tube (ETT) or tracheostomy tube for a minimum of 48 hours and admitted to Children's Hospital of Atlanta (CHOA) (Egleston/AMBH) NICU from 1/1/2020-12/31/2025. We plan to collect number of ETT/tracheostomy days, respiratory culture results, speciation of cultures, reported antibiotic sensitivities of collected specimens, duration and type of antibiotics administered, and total number of treatment days via REDCap database. From this data, we will calculate a baseline rate of tracheal aspirate cultures per 100 ETT/tracheostomy days and number of antibiotics per 100 ETT/tracheostomy days. This study has received CHOA Institutional Review Board approval. Results: CHOA specific data was obtained (in part) utilizing the Children's Hospitals Neonatal Database (CHND). A preliminary analysis of local data from the CHND revealed 759 infectious encounters in patients who met inclusion criteria. A manual chart review is currently underway. The most commonly isolated organisms were Pseudomonas (19%), MSSA (15%), Klebsiella (14%), Stenotrophomonas (11.7%), Serratia (8.2) and MRSA (4%). Further statistical analysis is pending. Conclusion: A thorough analysis of total infectious encounters, empiric antibiotic courses for presumed infections and the infectious organism trends in MV patients is anticipated to guide antibiotic stewardship, reduce number of MV and hospital days, and improve resource utilization. Subsequent QI projects will be guided by unit-specific diagnostic practices, antibiotic usage, and study data.

Reproductive, Adolescent & Women's Health

Heart Rate Variability and Quality of Life Outcomes Following a Biofeedback-Enhanced CBT Intervention for Adolescents with IBD

Shivaram, Swathi; Patrusky, Lauren; Ford, Ashley; Buzenski, Jessica; and Reed, Bonney

Presenting Author: Swathi Shivaram, BS (Clinical Research Coordinator)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 49

Abstract

Background: Adolescents with inflammatory bowel disease (IBD) experience reduced quality of life (QoL) and symptoms of autonomic dysfunction linked to anxiety and depression compared to healthy peers (Wang et al., 2025; Klages et al., 2020). Heart rate variability (HRV) biofeedback is a behavioral intervention targeting autonomic dysfunction associated with improvements in clinical outcomes related to QoL (Fournié et al., 2021). This study examined the associations between HRV metrics pre-and post-treatment with HRV biofeedback and QoL in adolescents with IBD receiving a biofeedback-enhanced cognitive behavioral therapy (CBT) intervention. Methods: 51 adolescents (ages 13–18) with IBD completed a 6-week manualized CBT intervention with HRV biofeedback. Adolescents and caregivers completed the Pediatric Quality of Life Inventory (PedsQL) at baseline and post-intervention. Adolescent resting HRV metrics (RMSSD, SDNN, NN, RR) were extracted using Kubios HRV Scientific software at baseline and post-intervention. Pre- and post-change scores of QoL and RMSSD were calculated. Positive change scores indicated improvement in autonomic functioning and QoL. Results: Paired T-tests revealed adolescent-reported QoL significantly improved from baseline to post-intervention ($p < .001$, 95% CI [3.89, 8.35]), whereas no significant difference was observed in parent-reported QoL. HRV indices did not significantly differ across treatment. A higher baseline RMSSD, reflecting better autonomic functioning, was positively associated with change scores indicating improvement in parent-reported QoL ($r = .38$, $p < .05$) but not adolescent-reported QoL. Baseline adolescent-reported QoL was positively associated with RMSSD change scores indicating improved autonomic functioning ($r = .39$, $p < .05$). Linear regression analysis indicated that better baseline autonomic functioning predicted improvement in parent-reported QoL ($b = .12$, $p < .05$), accounting for 12% of the variance. Conclusions: Adolescents with IBD demonstrated improved QoL following a 6-week CBT intervention. Although HRV did not significantly change across treatment, baseline autonomic regulation predicted parent-perceived improvement, suggesting HRV may reflect adolescents' capacity to engage with CBT skills. QoL was also associated with improved HRV, suggesting a potential bidirectional relationship that can be explored in future research. These findings support HRV as a potential predictor of treatment responsiveness rather than only a mechanism of change. Ongoing research is examining these relationships in a longer-duration intervention.

Gastrointestinal, Hepatic & Renal

Autoimmune Hepatitis and Rheumatologic Disease: An Underrecognized Challenge to Remission?

Solomon, Kristen MD; Rouster-Stevens, Kelly MD, PharmD; and Gupta, Nitika MD

Presenting Author: Kristen Solomon, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 14

Abstract

Background:Autoimmune hepatitis (AIH) is a rare progressive inflammatory liver disease that can progress to end-stage liver disease without treatment. The goal of therapy is biochemical remission within 6 months of treatment initiation. Standard therapy with corticosteroids alone or in combination with azathioprine achieves remission in 75–80% of patients, though outcomes are worse in certain patient populations. AIH frequently coexists with other autoimmune diseases, with 30–50% of patients having concurrent autoimmune conditions. We sought to determine whether coexisting rheumatologic disease increases the risk of refractory AIH and worse prognosis, and to identify criteria to help recognize patients at high risk of refractory disease.Methods: A retrospective review of patients with AIH who attended hepatology clinic at Children's Healthcare of Atlanta between 2014 and 2021 was conducted. We identified 71 patients with autoimmune hepatitis type 1 or type 2 based on biochemical testing or pathology using the International Autoimmune Hepatitis Group (IAHG) Scoring Criteria. Baseline demographics were collected, and clinical and biochemical factors were assessed to identify patients with coexisting rheumatologic conditions, with outcomes compared to patients with AIH who had negative rheumatologic screening.Results: Seventy-one pediatric patients with autoimmune hepatitis (AIH) were identified, predominantly female and of Black race, with a median diagnosis age of 12 years. Sixty-nine percent underwent rheumatologic evaluation, and nine patients, all female, had confirmed rheumatologic disease. Refractory AIH occurred in 89% of rheumatologic-positive patients versus 37.5% of control patients. The rheumatologic-positive cohort had a higher frequency of liver transplantation or listing and a younger median age at diagnosis.Conclusion Patients with coexisting rheumatologic disease had a younger median age at diagnosis, more refractory AIH, and increased incidence of liver transplant or death. We postulate that coexisting rheumatologic disease confers higher risk of refractory disease and worse clinical outcomes. Future multicenter studies are needed to define clinical phenotype, establish comprehensive screening protocols, and implement personalized therapies to improve outcomes.

Data Science, Methods & Biomarkers

A Simple Approach Toward Quantifying Extracellular Vesicles From Various Cells of Origin in Complex Biofluids

Padmanabhan, Samhita; Srinivasan, Advika; Kingwell, Emma; Kumar, Prashant; and Tirouvanziam, Rabindra

Presenting Author: Advika Srinivasan, MS (PhD Student)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16501 and 16502
Poster Number: 84

Abstract

Background: Extracellular vesicles (EVs) are ubiquitous membrane-bound messengers carrying nucleic acids, proteins, and metabolites between cells. EVs accumulate in large amounts (billions to trillions) in biofluids, and it is key to determine not only their content, but also their cell of origin, to better understand their signaling impact in health and disease. In particular, our lab has demonstrated a key role for airway EVs in mediating chronic inflammation and bacteria tolerance in cystic fibrosis (CF) patients. Shotgun proteomics have been used in the past to infer the proportion of EVs from various sources in complex biofluids, however, proteins can be bound to the surface of EVs extracellularly (post-secretion), making this method faulty. Methods: EVs were isolated by differential centrifugation and tangential flow filtration with a 300 kDa filter from CF sputum (mixed origin) and cultures of epithelial cells, macrophages and neutrophils (single origin). Magnetic beads conjugated to biotinylated antibodies against cell-specific surface markers (CD66b, CD115 and CD326 for neutrophils, macrophages and epithelial cells, respectively) were used for EV pull-down. Counting and physical properties of EVs (whole and sorted fractions) were then quantified by nanoparticle tracking analysis (Nanosight). Results: The approach delineated above enables efficient sorting of EVs by cell of origin, with relative proportions of EVs from different subsets adding up to the total count of unseparated EVs. CF sputum demonstrated a predominance of neutrophil-derived EVs (>75%). Total EV count and proportions based on cell of origin were dependent on disease severity across patients, likely reflecting differences in the nature of airway inflammation at various stages of CF. Conclusions: This study establishes a simple and reproducible approach for separating EVs from complex biofluids such as CF sputum using transmembrane protein markers specific for given cell subsets. Neutrophil-derived EVs represented the largest proportion of total EVs in CF sputum, consistent with our prior studies establishing a key role for these cells in pathological signaling within CF airways. The approach outlined here enables to probe cell-specific contributions to EVs in biofluids (e.g., sputum or blood) and their potential immunomodulatory and tissue remodeling functions across pediatric and adult diseases.

Neurodevelopment & Autism Interventions

Congenital Myasthenic Syndrome Due to Novel GFPT1 Variant Presenting with Head Drop and Visual Impairment: A Case Report

Torrey, Katharine; Tiongson, Emmanuelle; Logan, Rachel; Dennison, John; Fischer, Andrew; Jain, Nieraj; Schniederjan, Matthew; Maselli, Ricardo; Verma, Sumit

Presenting Author: Katharine Torrey, BA (MD Student)

Poster Information: Afternoon (4:45 pm - 5:45 pm), 16th Floor, CL. 16501 and 16502
Poster Number: 92

Abstract

Background: Glutamine-fructose-6-phosphate transaminase 1 (GFPT1) gene variants are a known cause of congenital myasthenic syndrome (CMS), typically presenting with fatigable limb-girdle weakness and characteristic tubular aggregates on muscle biopsy, though recent reports have broadened the clinical spectrum. Methods: We describe a 5-year-old boy presenting with hypotonia, progressive muscle weakness with head drop, cognitive delay, and visual impairment. Neuromuscular genetic panel revealed a novel homozygous missense variant in GFPT1 (c.1154G>A, p.R385Q). Results: Further workup showed white matter abnormalities with atrophy of corpus callosum and cerebellum on neuroimaging, myopathic motor unit potentials on electromyography, and non-specific changes on the left biceps muscle biopsy with absence of tubular aggregates. Stimulated jitter analysis of the right orbicularis oculi muscle showed increased jitter and blocking indicative of neuromuscular junction (NMJ) transmission defect. The whole-exome sequencing confirmed autosomal recessive GFPT1-related myasthenia [p.(Arg367Gln) (CGG>CAG): c.1100 G>A in exon 12 of the GFPT1 gene (NM_002056.3)]. Proband's parents were heterozygous for the p.(R367Q) variant in the GFPT1 gene and in silico analysis predicted a deleterious impact on protein structured and function, classifying this novel variant as likely pathogenic. Conclusions: The muscle-eye-brain (MEB) phenotype of our case was like that of alpha-dystroglycanopathy, a glycosylation-related severe congenital muscular dystrophy. Additionally, the presence of NMJ abnormalities expands the novel GFPT1 variant genotype-phenotype to include muscle-eye-brain congenital myasthenic syndrome (MEB-CMS). This case study highlights the role of multiple specialists (neurologist, radiologist, ophthalmologist, pathologist), and neurodiagnostic techniques (neuroimaging, electrodiagnostic, muscle pathology) to deep phenotype symptomatic patients with novel gene variants found on next-generation sequencing.

Reproductive, Adolescent & Women's Health

Emotional Abuse and Neglect as Predictors of Emotion Regulation Difficulties and Depressive Symptoms in Adolescents

Torrey, Katharine; Sato, Adrienne; Bloemker, Malia; and LoPilato, Allison.

Presenting Author: Katharine Torrey, BA (MD Student)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16503 and 16504
Poster Number: 50

Abstract

Background: Early-life exposure to trauma is linked to increased risk of psychiatric disorders. Difficulties in emotional regulation are linked to both childhood trauma and mental illness and are associated with poorer clinical outcomes, including reduced responsiveness to psychiatric treatments. This project examined the role of traumatic childhood experiences on emotional regulation and depressive symptoms in adolescents recruited through the Emory Child and Adolescent Mood Program. Understanding the role of emotion regulation may inform intervention strategies to reduce long-term mental health risk among trauma-exposed youth through emotion-regulation targeted treatments.Methods: Participants (N = 105; ages 14-17; M = 15.43) included adolescents meeting DSM-5 criteria for a depressive and/or anxiety disorder (n = 76) and healthy controls with no lifetime psychiatric diagnoses (n = 29). Self-report measures included the Childhood Trauma Questionnaire Short Form (CTQ-SF), Difficulties in Emotion Regulation Scale Short Form (DERS-SF), and Beck Depression Inventory (BDI-II). Pearson correlations examined associations among childhood maltreatment (CTQ subscales), emotion dysregulation (DERS), and depressive symptoms (BDI). Results: Emotion dysregulation was significantly associated with emotional abuse (r = .46), sexual abuse (r = .28), emotional neglect (r = .38), and physical neglect (r = .21), but not physical abuse. Depressive symptoms were significantly correlated with emotional abuse (r = .56), sexual abuse (r = .33), emotional neglect (r = .41), physical neglect (r = .22), and DERS scores (r = .75). In regression analyses, emotional abuse (B = .290, **β** = .313) and emotional neglect (B = .288, **β** = .348) uniquely predicted emotion regulation difficulties, while emotional abuse (B = 1.094, **β** = .415), emotional neglect (B = .884, **β** = .376) and physical neglect (B = –1.212, **β** = –.285) independently predicted depressive symptom severity. All p values were <0.05. Conclusions:Findings suggest that exposure to childhood maltreatment is associated with clinically meaningful disruptions in emotion regulation during adolescence. Emotional abuse and emotional neglect were particularly strong predictors of emotion dysregulation, indicating that specific trauma subtypes may confer differential risk for emotion regulation impairments and depressive symptom severity. Assessment of trauma history and emotion regulation functioning may facilitate early risk identification and inform targeted, prevention-oriented interventions focused on emotion regulation to reduce long-term psychiatric morbidity among youth who have experienced trauma.

Neurodevelopment & Autism Interventions

Visual Memory Strengths and Verbal Recall Challenges in Children: Insights from EPMLT

Tuey, Mitchell; Shade, Taylor; Swinehart, Lydia; Okada, Noah; Ammons, Carla; Winston, Molly; Bearden, Don; Gershon, Tim; and Drane, Daniel

Presenting Author: Mitchell Tuey, BS (Undergraduate student)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 22

Abstract

Episodic memory develops unevenly across childhood, with visual recognition skills typically emerging sooner than verbal learning abilities. Recent developmental research indicates that children rely heavily on perceptual detail when encoding events, as visual memory networks mature earlier and support strong recognition performance throughout childhood (Riggins, 2015). In contrast, verbal recall relies on linguistic and associative systems that are developed later in life. Naturalistic learning tasks highlight these differences more clearly than traditional list-based memory assessments, as children tend to remember visual scenes and contextual details far more effectively than arbitrary labels (Bauer, 2015). The Emory Pediatric Multimodal Learning Test (EPMLT) provides an opportunity to examine these developmental patterns using video episodes that require children to recall names, visual content, and spatial information. To investigate these domain differences, the EPMLT was administered to eleven pediatric participants. Data was analyzed across verbal recall tasks (e.g., character names) and visual/content tasks (e.g., identifying actions and locations). Percent accuracy was calculated for each domain based on total points possible. Across participants, visual and content-based memory outperformed verbal recall. Many patients scored between 40-60% on content and spatial tasks, consistent with evidence that scene memory and spatial encoding are early-emerging strengths that remain relatively stable across development. On the other hand, verbal name recall rarely exceeded 30% and frequently reached zero, mirroring research showing that children's lexical retrieval, especially for proper nouns, is highly resource-dependent and not yet fully efficient until 15-17 years old (Wojcik, 2013). Overall, these results suggest that when children learn from naturalistic scenes, they rely substantially more on visual and contextual information than on verbal layers. The EPMLT captures these developmental differences effectively and may offer a more ecologically valid framework than previous tests for assessing pediatric memory. Future works may examine whether these domain-specific patterns can predict long-term retention or identify cognitive subprofiles in pediatric populations.

Oncology & Cellular Therapy

Patterns of Neuropathy Trajectory During Therapy for Childhood Acute Lymphoblastic Leukemia

Vajdic, Tyler MD,MS; Lee, Katherine MPH; Stevenson, Jason MD, MSCR; Porter, Chris MD; Khanna, Anjali MBBS, MPH; Hawk, Ashleigh MPH; Huerta, Jazmin MPH; Alao, Oyin MPH; Byers, Elizabeth MD; Kuhn, Amanda MD; Bergsagel, John MD; Castellino, Sharon M. MD, MSc; and Miller, Tamara P. MD, MSCE

Presenting Author: Tyler Vajdic, MD, MS (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 29

Abstract

Background: Vincristine (VCR), a key component of chemotherapy for pediatric acute lymphoblastic leukemia (ALL), frequently causes vincristine-induced peripheral neuropathy (VIPN). Although persistent VIPN after therapy has been well described, few studies have evaluated how VIPN evolves over the course of treatment. Methods: This retrospective cohort included children aged 1–21 years with ALL treated with standard chemotherapy courses (induction, consolidation, delayed intensification, interim maintenance, +/- interim maintenance 2, maintenance) at Children's Healthcare of Atlanta from 2010-2022. VIPN incidence, time from ALL diagnosis, and outcomes (duration, time to pharmacologic treatment, and time to symptom resolution) were manually abstracted from the electronic health record using a standardized chart review process. Persistent neuropathy was defined as symptoms extending beyond ALL treatment completion. Analyses were performed in RStudio using Pearson's chi-squared test, Fisher's exact test, and one-way analysis of means. Results: Among 414 eligible patients, 308 (74%) experienced at least one VIPN adverse event (AE). Sixty patients experienced multiple episodes, yielding 374 total neuropathy AEs. Excluding induction, consolidation (N=55/170, 32%) and maintenance (N=60/198, 30%) had the highest frequencies of incident neuropathy AEs. The most common trajectory was neuropathy onset and resolution during therapy without recurrence (N=162/308, 53%). Persistent neuropathy occurred in 117 (38%) patients, while 28 (9%) experienced recurrent neuropathy that resolved before therapy completion. Median time from ALL diagnosis to neuropathy was 75 days (Interquartile range (IQR): 25-209 days). Median neuropathy duration was 197 days (IQR: 64-479 days). Neuropathy-directed pharmacotherapy was initiated in 141 patients (46%) at a median of 19 days (IQR: 0-187 days) following symptom onset. Conclusion: VIPN is highly prevalent during ALL therapy, and more than one-third of patients with ALL experience symptoms beyond completion of upfront therapy. Even when neuropathy resolves during therapy, symptom burden is prolonged, with a median duration exceeding 6 months. These findings have important implications for patient counseling and highlight the need for early identification and optimized management strategies. Ongoing analyses will assess the comparative effectiveness of neuropathy-directed interventions and the impact of VCR dose modifications on neuropathy trajectories.

Cardiac Bioengineering & Platforms

Evaluation of Intermediate Cardiac MRI Findings in Children and Young Adults with Acute Myocarditis

Videlefsky, Karin; Puckett, Rachel; Wilson, Hunter C.

Presenting Author: Karin Videlefsky, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 6

Abstract

Background: Cardiac magnetic resonance (CMR) is frequently used to guide management in children and young adults diagnosed with acute myocarditis. However, there are limited data on how CMR findings evolve after initial presentation.Objective: We aimed to: 1) compare baseline and follow up CMR findings in children and young adults with myocarditis; and, 2) identify differences in baseline features of patients who had resolution of late gadolinium enhancement (LGE) on follow-up CMR compared to those who did not. Methods: We included patients <22 years old at our center diagnosed with myocarditis between 9/1/20 and 9/1/25 who had both initial and follow-up CMR. We excluded patients with significant congenital heart disease and with genetic or clinical findings concerning for cardiomyopathy. Clinical, laboratory, and imaging data were collected. Groups were compared using standard univariate tests. Results: 44 patients were identified. Patients were median 16.9 (interquartile range [IQR] 15.3, 17.4) years old, mostly male (89%), and had median hospital length of stay of 3 (IQR 2,4) days. Median time between initial and follow-up CMR was 112.0 (IQR 94.5, 168.0) days. There was a significant reduction in pre-contrast T1 times (indicator of fibrosis/edema) and T2 times (indicator of edema) on follow-up CMR. LGE persisted in 30/44 (68%) of patients. There were no significant differences in age, baseline maximum troponin, ejection fractions, or T1 and T2 times at baseline between groups with and without LGE resolution on follow-up CMR (Table). However, patients with persistent LGE on follow-up CMR had an increased number of myocardial segments with LGE on baseline CMR compared to patients with LGE resolution (5.0 vs 2.0 myocardial segments with LGE, p=0.03).Conclusions: Intermediate follow-up CMR after diagnosis of myocarditis showed improvement in pre-contrast measures of inflammation and edema. There was persistent LGE in most cases which was associated with greater number of segments with LGE on baseline CMR. Findings may provide clinicians guidance in timing of and expectations for follow-up CMR in patients seen in clinic after diagnosis of acute myocarditis.

Pulmonary, Respiratory Support & Cystic Fibrosis

Making sense out of nonsense: Rescuing cystic fibrosis-causing variants lost in translation

Winters, Ashlyn G.; Freestone, Emily; Yang, Yolanda; White, Janiyah; Jackson, JaNise J.; Foye, Catherine; Lopes-Pacheco, Miquéias; Hartman IV, John L.; Sorscher, Eric J.; and Oliver, Kathryn E.

Presenting Author: Ashlyn Winters, BS (Research Specialist)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 34

Abstract

Background Cystic fibrosis (CF) is a lethal autosomal recessive disorder caused by mutation of the CF transmembrane conductance regulator (CFTR). Gene variants confer defects in the encoded CFTR epithelial ion channel, together with pathological findings in numerous tissues that to date, have not been completely restored by clinically approved drugs (i.e. "CFTR modulators"). Among the U.S. CF population, ~13% of patients carry premature termination codons (PTCs) – otherwise termed "nonsense" variants – such as G542X and W1282X. PTCs result in early degradation of encoded transcripts and proteins, the consequences of which are associated with severe disease phenotypes. Moreover, PTCs are largely off-label for CFTR modulators. Our goal is to address unmet needs through development of genetic based interventions that rescue synthesis of enzymatically active, full-length CFTR. Methods We previously identified gene-gene interaction networks implicated in read-through of CF-causing nonsense variants. In particular, genetic disruption of ribosomal proteins L8 (RPL8) and L12 (RPL12) significantly improved biogenesis of G542X- and/or W1282X-CFTR. In the present study, mechanisms by which RPL depletion impact PTC processing were pursued, including tests of additivity/synergy with CFTR modulators and other compounds. RPLs were suppressed ~50% by siRNA knockdown in Fischer rat thyroid cells, an established CF model. Cells were stably transduced with G542X or W1282X cDNA, as well as a luminescent reporter to measure plasma membrane localization. CFTR mRNA and protein expression were also monitored. Results Cell surface trafficking of G542X and W1282X was strongly increased by RPL8 or RPL12 knockdown, and synergistic enhancement was observed with the combination of G418 (read-through agent), PTI-428 (mRNA amplifier), or elexacaftor-tezacaftor-ivacaftor (CFTR modulators). RT-qPCR and western blots indicate G542X and W1282X mRNA and protein levels were significantly augmented by siRPL8, either alone or with concomitant G418 or PTI-428. Conclusions Our findings suggest that partial depletion of RPL8 or RPL12 impairs mRNA decay and confers PTC read-through to amplify G542X and W1282X synthesis. Tuning expression of these RPLs should be considered as a novel therapeutic strategy for CFTR nonsense suppression – the effects of which may be augmented in a multiplicative manner by clinically approved drugs – thereby potentially benefitting people living with otherwise untreatable variants.

Oncology & Cellular Therapy

Procedural Sedation Outside the Operating Room in Pediatric Hematopoietic Stem Cell Transplant Patients

Xiao, Heather; Burger, Rebecca; and Kamat, Pradip

Presenting Author: Heather Xiao, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 30

Abstract

Background: Patients with hematopoietic stem cell transplantation (HCT) frequently require procedural sedation (PS) for diagnostic and therapeutic procedures and often have comorbidities that may increase the risk of sedation-related adverse events (AEs). The study objective was to compare the incidence of AEs and interventions during PS performed outside the operating room (OR) in patients pre- and post-HCT.Patients And Methods: This was a single-center retrospective case-control study of all patients with HCT who had PS outside the OR between January 2015 and January 2025. Sedation encounters were matched by the same procedure before and after HCT, and AEs were analyzed using a paired case–control design and generalized linear mixed models.Results: Of the 118 total patients, there were 332 sedation encounters. The median age in years was 7.6 (IQR 3.5-11.9) and 42.4% patients were female. Procedures included: bone marrow biopsy/bone marrow aspiration (66%), lumbar puncture (9%), magnetic resonance imaging (10.8%), and nuclear medicine imaging (7.8%), with 36.7% undergoing two procedures during a single sedation encounter. A combination of propofol and fentanyl was used in 75.3% of these sedation encounters. Hypoxia was the most frequent low-high-risk AE both pre and post HCT (11.4% and 20.5% respectively). Blow by oxygen was the most common intervention and was required significantly more frequently post HCT (10.2% vs 25.3% respectively, $p<0.001$). Only one sedation failure was noted, and there were no cardiac arrests, aspiration events, or deaths.Conclusion: Procedural sedation performed outside the operating room was not associated with any high-risk or critical adverse events in post-HCT patients compared to patients pre-HCT. Although the cohort size was limited, these findings suggest an important opportunity to minimize unnecessary exposure to general anesthesia and to optimize resource allocation.

Gastrointestinal, Hepatic & Renal

Contrast-enhanced Colosonography for Evaluation of Anorectal Malformations in Children

Xu, Hongmin; Gagnon, Marie-Helene; Ali, Sumera; and Linam, Leann.

Presenting Author: Hongmin Xu, MD (Medical Fellow)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16403 and 16404
Poster Number: 15

Abstract

Background: The purpose of this clinical research abstract is to: 1. Review the embryology, classification, and imaging features of Anorectal malformations (ARMs). 2. Review the indications and techniques of Contrast-enhanced colosonography (CeCS) in pediatric patients with ARMs. 3. Provide sample cases and clinical courses of patients with specific ARMs. Anorectal malformations (ARMs) are a spectrum of congenital anomalies involving abnormal development of the distal rectum and anus, leading to an interruption or misconnection of the normal passage between the rectum and the perineum. The condition occurs in about 1 in 5,000 live births and can range from mild to complex. Children with ARMs require preoperative imaging to assess the presence and location of a fistula. Identifying the location of these fistulas is crucial for surgical planning. Contrast-enhanced colosonography (ceCS) has emerged as a valuable tool in diagnosing pediatric ARMs, offering superior anatomical visualization and assessment compared to traditional imaging techniques like fluoroscopy, providing more sensitive and specific evaluation of fistula with real-time contrast filling. CeCS avoids ionizing radiation, and ultrasound contrast agents have a high safety profile with low incidence of adverse events compared to other contrast agents. Methods:CeCS technique should include survey greyscale ultrasound examination of the pelvis to assess for adequate window. Lumason, the ultrasound contrast agent, is prepared and injected into a bag of saline, with a ratio of 1:500mL. The mucous fistula is examined, and a small Foley catheter is inserted by the radiologist, then balloon is inflated to create a seal. Contrast is then instilled by gravity drip, and perineal structures are imaged through three standard approaches-transabdominal, transperineal and posterior sagittal approaches. As well as reviewing the topics above, this review will also provide examples of ARM cases demonstrating high, intermediate, and low fistulas seen on CeCS. Results:Compared with Fluoroscopy, Contrast-Enhanced colosonography has higher sensitivity and specificity to evaluate fistulas associated with anorectal malformations.Conclusions:Overall, CeCS enhances the diagnostic accuracy and management of pediatric ARMs. By providing detailed anatomical insights and allowing for accurate classification, it plays a critical role in guiding treatment strategies and improving long-term outcomes for affected patients.

Public Health, Equity & Care Delivery

Pulmonary Artery Pulsatility Index Score in the Adult Congenital Heart Disease Population: A Hemodynamic Marker of Significance?

Arslan Zahid, MD; Mollie Westrick, BA Fred Rodriguez III, MD; and R. Allen Ligon, MD

Presenting Author: Arslan Zahid, MD (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 38

Abstract

Background: The pulmonary artery pulsatility index (PAPi) is a validated marker of right ventricular dysfunction. The utility of this index has been studied in predicting mortality in adults with acute pulmonary embolism, pulmonary arterial hypertension, and advanced heart failure. For the latter population, this index marker predicts the need for hospitalization and/or need for advanced therapies. Currently, there is no literature surrounding the utility of the PAPi in the adult congenital heart disease (ACHD) population. The objectives of this study were to calculate PAPi in an ACHD catheterization database and to determine whether an abnormal PAPi score could help risk stratify ACHD patients. Methods: A retrospective review was performed of ACHD patients who underwent cardiac catheterization at a large volume tertiary care facility from August 2023 to January 2025. Patients with single ventricle physiology were excluded due to inability to calculate the PAPi. Patients were stratified based on PAPi value with evidence-based cutoffs of 0.9, 1.85, and 3.9 to differentiate abnormal values extrapolated from the use of these scores to predict adverse outcomes in patients at risk for right ventricular failure. Biventricular circulation patients with a systemic right ventricle after atrial switch were calculated in the same format with atrial limb pressure used as the right atrial pressure. Primary outcomes included all-cause mortality, hospitalization in the follow-up period, need for additional procedural cardiac intervention in the follow-up period, and occurrence of any major adverse cardiac event (MACE). Fisher's exact test was used to compare patients with respect to the PAPi cutoff against clinical outcomes; significance level set at 0.05. Results: 66 patients met study criteria. The median study follow up period was 1 year. Using PAPi cutoffs of 0.9, 1.85, and 3.9 respectively, there was no significant difference in all-cause mortality (p=0.61; p=0.71; p=0.55), hospitalization in the follow-up period (p=1.0; p=0.31; p=0.67), need for additional cardiac procedural intervention (p=0.48; p=0.11; p=0.37), or MACE (p=0.70; p=0.77; p=0.13) Conclusion: Calculation of a PAPi was not statistically significant marker for predicting clinical outcomes such as hospitalization, need for intervention, or MACE.

Public Health, Equity & Care Delivery

School-Based Mental and Behavioral Health Referrals to the Pediatric Emergency Department

Zwiebel, Hannah; Haber, Rebecca; Harris, Julia; and Okeson, Karli

Presenting Author: Hannah Zwiebel, MD, MPH (Medical Resident)

Poster Information: Morning (8 am - 9 am), 16th Floor, CL. 16501 and 16502
Poster Number: 39

Abstract

Background: There is an increase in patients who presents to the pediatric emergency department (PED) for Behavioral Mental Health (BMH) concerns, and schools are in a unique position to address needs in the spectrum of pediatric mental health care. The purpose of this study is to examine the population of patients who are referred to the PED for behavioral and mental health concerns by school personnel compared to other referral sources. Methods: This study is a retrospective chart review of visits to the PED at a large academic pediatric hospital between July 2022 – June 2024. Patients who visited during these dates for a chief complaint of "behavioral complaint" of any gender, ages 10-17 were eligible to be included into the study. Data was collected related to demographics, medical history, and the emergency department visit. Preliminary statistical analysis included medians, Wilcoxon rank sum tests, Fisher's exact tests, and Pearson's Chi-squared tests. Results: Data was collected from 430 PED visits. 181 patients were referred from school (42%). There was a significant number of patients who presented to the PED for suicidal ideations from schools (65%) vs other referral sources (46%), ($p<0.001$). For aggressive behavior, 21% were school-based referrals and 40% of patients were from all other referral sources ($p<0.001$). There was no difference between groups on whether a psychiatric hold was signed [school-82/181 (45%), other source-114/249 (46%), $p=0.862$)] Conclusions: Patients who are referred from schools to the PED for BMH complaints are more likely to present with suicidal ideations. There were significantly less referrals from schools for aggressive behavior. Need for psychiatric hold was not significantly different between those referred from school vs other referral sources. This may indicate that schools have resources to identify suicidal emergencies, and perhaps that schools have resources to handle aggressive outbursts. Data analysis is ongoing.

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