CURRICULUM VITAE

Nicole Mayer Hamblett, PhD

EDUCATION

1990-1994	B.S. 1994, Department of Mathematics, Santa Clara University, Santa Clara, CA, *Cum laude*
1994-1996	M.S. 1996, Department of Biostatistics, School of Public Health, University of Washington, Seattle, WA
1996-1999	Ph.D. 1999, Department of Biostatistics, School of Public Health, University of Washington, Seattle, WA

FACULTY POSITIONS

2000-2004	Instructor, Department of Pediatrics, School of Medicine, University of Washington, Seattle, WA
2004-2010	Research Assistant Professor, Department of Pediatrics, School of Medicine, University of Washington, Seattle, WA
2010-2012	Research Associate Professor, Department of Pediatrics, School of Medicine, University of Washington, Seattle, WA
2012-2017	Associate Professor, Department of Pediatrics, School of Medicine, University of Washington, Seattle, WA
2013-2017	Adjunct Associate Professor, Department of Biostatistics, School of Public Health, University of Washington, Seattle, WA
2017-present	Professor, Department of Pediatrics, School of Medicine, University of Washington, Seattle, WA
2017-present	Adjunct Professor, Department of Biostatistics, School of Public Health, University of Washington, Seattle, WA

HOSPITAL POSITIONS

2020-2022	Interim Co-Director, Center for Clinical and Translational Research, Seattle Children's Research Institute

HONORS, AWARDS, AND SCHOLARSHIPS

2024	Honorary Chair, Cystic Fibrosis North American Conference, Boston, MA

BOARD CERTIFICATION

Not Applicable

CURRENT LICENSES

Not Applicable

PROFESSIONAL ORGANIZATIONS

1998-present American Statistical Association

TEACHING RESPONSIBILITIES

1994	Teaching Assistant, Medical Biometry I, Department of Biostatistics, University of Washington. (Lectured one section per week, held office hours, graded homework, introduced and taught SPSS).
1995-1996	Teaching Assistant, Introductory Biostatistics I and II, Department of Biostatistics, University of Washington, Seattle, WA. (Held office hours, graded homework, lectured, introduced and taught Splus).
2000-2003	Co-Instructor, "Epidemiology as a Research Tool in CF," North American Cystic Fibrosis Conference. (Short course, taught bi-annually)
2001-2003	Co-Instructor, "Design of Clinical Research Study," Seattle Children's Hospital. (Three session course, taught annually).
2003	Guest Instructor, "Interpretation of Adverse Events in Pediatric Clinical Trials: Signal or Noise?", Practical Aspects of Conducting Pediatric Clinical Research Trials, Seattle Children's Hospital.
2002-2004	Guest Instructor, "Considerations when Designing Clinical Trials for Cystic Fibrosis," Department of Biostatistics Lectures in Applied Statistics Course, University of Washington, Seattle, WA. (One lecture taught annually).
2006-2009	Instructor, "Statistics for Laboratory Research," Short Course for Pediatric Fellows and Faculty at Seattle Children's Hospital
2008	Guest Instructor, "Bioinformatics in the Clinical Trial Setting", Translational Informatics Research Seminar, University of Washington.
2012-2013	Instructor, Core for Biomedical Statistics Lecture Series, Seattle Children's Hospital

Graduate Research Assistant Mentoring:

2001-2002	Jon Campbell, Dept. of Biostatistics, University of Washington
2003-2005	David Coblentz, Dept. of Biostatistics, University of Washington
2007-2009	Paramita Saha, Dept. of Biostatistics, University of Washington
2005-2006	Umer Khan, Dept. of Biostatistics, University of Washington
2006-2007	Margaret Kloster, Dept. of Biostatistics, University of Washington
2011-2012	Laina Mercer, Dept. of Biostatistics, University of Washington
2011-2013	Leigh Fisher, Dept. of Biostatistics, University of Washington
2016-2020	Renee Russell, Dept. of Biostatistics, University of Washington
2018-2020	Xinyun Dai, Dept of Biostatistics, University of Washington
2018-2024	Mark Warden, Dept. of Epidemiology, University of Washington

Academic Advisor:

2006-2008 Tracy Bartz, Dept. of Biostatistics, University of Washington

Dissertation Committee:

2014-2016 Leila Zelnick, Department of Biostatistics, University of Washington

2017-2018 Maria Nelson, Department of Microbiology, University of Washington

Select Research Scientist Mentoring:

2009-2019 Sonya Heltshe, PhD; faculty member in the University of Washington Department of Pediatrics
2010-2019 Tamara Simon, MD; NIH K-12 mentoring committee member
2011-2013 Bradley Quon, MD; MPH student and junior CF researcher
2012-2014 Evelyn Hsu, MD; Seattle Children's Hospital Clinical Research Scholar Program
2014-present Kathleen J. Ramos, MD; Fellow, Dept. of Medicine, University of Washington
2014-2016 Matthew Crull, MD; Fellow, Pulmonary and Critical Care Medicine, University of Washington
2015-2017 Kelly Evans, MD; Seattle Children's Clinical Research Scholar Program
2015-2018 Dale Lee, MD, Seattle Children's Clinical Research Scholar Program
2016-2022 Ranjani Somayaji, MD, MPH, Assistant Professor, University of Calgary
2022-2023 Omar Bayomy, MD, Fellow, Dept of Medicine, University of Washington
2023-present Oliver McElvaney, MD, PhD, Fellow, Dept of Medicine, University of Washington

EDITORIAL RESPONSIBILITIES

2015-2017 Statistical Editor, *Thorax*

SPECIAL NATIONAL RESPONSIBILITIES

2014-present Co-Executive Director, Cystic Fibrosis Therapeutics Development Network Coordinating Center, Seattle Children's Research Institute, Seattle, WA

Cystic Fibrosis Foundation:

2000-present Member, CF-TDN Steering Committee
2009-2012 Member, CF-TDN Translational Center Committee
2010-present Member, Cystic Fibrosis Foundation (CFF) Biomarker Consortium
2010-present Member, CFF Clinical Research Executive Committee
2012-present Member, CFF National Patient Registry Committee
2016-present Member, CFF Clinical Research Advisory Board
2021-present Member, CF Genetic Therapy Working Group

National Institutes of Health:

2005-2009 Member, Steering Committee for the NHLBI funded Early *Pseudomonas* Infection Control Trial (EPIC)
2011 Participant, NHLBI Best Practices in Data Coordinating Centers Workshop
2021-2023 DSMB Member, NIAID

Food and Drug Administration:

2005	Participant, FDA/CFF working group "Patient Reported Outcome Measure Development in Cystic Fibrosis"
2010	Participant, FDA meeting "Cystic Fibrosis Clinical Research: Key Issues Related to the Efficient Conduct of Clinical Trials Supporting Registration of Investigational New Drugs"
2012	Invited Lecturer, Office of Biostatistics, "Understanding and Optimizing the Pulmonary Exacerbation Endpoint: Preparing for the Next Generation of Cystic Fibrosis Clinical Trials"
2012	Invited Panelist, "Issues in the Design of Clinical Trials for Antibacterial Drugs for the Treatment of Non-CF Bronchiectasis", FDA Public Workshop, Bethesda, MD
2014	Invited Panelist, "Complex Issues in Developing Drug and Biologic Products for Rare Diseases", FDA Public Workshop, Silver Springs, MD
2014-present	Special Government Employee (SGE), Advisory Committee Member
2023-present	Advisory Committee Member, Pulmonary-Allergy Division

Institute of Medicine:

2013	Invited Panelist, Committee on Strategies for Responsible Sharing of Clinical Trial Data, National Academy of Science, Washington D.C.

National Academy of Sciences

2024	Invited Contributor, Nattional Academy Meetings on Rare Diseases

International Grant Reviewer Responsibilities:

Ireland Health Research, German Ministry of Education, United Kingdom Medical Research Council

SPECIAL LOCAL RESPONSIBILITIES

1999-2005	Manager, Statistical Analysis Unit, Cystic Fibrosis Therapeutics Development Network Coordinating Center
2000-present	Co-organizer, Cystic Fibrosis Biostatistics and Epidemiology Working Group, University of Washington, Seattle, WA
2002-2004	Member, Steering Committee for the NIH National Center for Research Resources (NCRR) Grant
2005-2010	Member, Scientific Advisory Committee, Seattle Children's Hospital
2005-2007	Associate Director, Biostatistics, Cystic Fibrosis Therapeutics Development Network Coordinating Center
2007-2011	Director, Biostatistics and Clinical Data Management, Cystic Fibrosis Therapeutics Development Network Coordinating Center
2010-2012	Acting Director, Seattle Children's Research Institute Core for Biomedical Statistics
2011-2014	Co-Director, Cystic Fibrosis Therapeutics Development Network Coordinating Center, Seattle, WA
2011-present	Member Center for Clinical and Translational Research Executive Committee

2011-present Faculty Mentor, University of Washington Department of Pediatrics, Clinical Research Scholar Program
2012-2018 Co-Director, Seattle Children's Research Institute Core for Biomedical Statistics
2013-2016 Member, Institutional Review Board

Grant Reviewer:
Department of Pediatrics Academic Enrichment Fund Grants
Core for Clinical and Translational Research Pilot Grants
Department of Pediatrics Clinical Research Scholar Program Grants
Institute for Translational Health Sciences Pilot Grants

CURRENT FUNDING

As Principal Investigator:

HAMBLE24A0-AP 12/01/2023-11/30/2030 3.6 Calendar Mo.
Cystic Fibrosis Foundation $13,952,674
REACH: A Research Study to Advance the CF Therapeutics Pipeline for People without Modulators
The objectives of this grant are to develop a regulatory compliant trial-opportunity communication platform to promote research engagement in pwCF not eligible for HEMT, conduct a prospective, longitudinal, "light touch" research study to obtain key research quality CF outcome data, conduct sub-studies to obtain specialized outcome data to inform the evaluation of nucleic acid-based therapies (NABTs) and promote innovative trial designs with the use of shared control data.
Role: PI

MAYFLOWER-HAMBLE21A0-P 1/1/2021-12/312/27 0.3 Calendar Mo.
Cystic Fibrosis Foundation $6,040,754
Material and Fetal Outcomes in Era of Modulators
The objectives of this grant are to characterize changes in FEV1 over the course of pregnancy based on cumulative CFTR modulator use while pregnant along with defining other factors that may influence changes in pulmonary function including duration of pre-pregnancy modulator use, baseline FEV1, genotype, history of exacerbations, and pre-existing co-morbid conditions.
Role: Coordinating Center PI

HAMBLE22K0 6/1/2022-5/31/2026 0.12 Calendar Mo.
Cystic Fibrosis Foundation $5,154,261
Prospective Evaluation of a Standardized Approach to Diagnosis (PREDICT) & Treatment (PATIENCE) of Nontuberculous Mycobacteria Disease in CF (NTM-OB-17)
The study will help define a standardized approach to the diagnosis and treatment of NTM pulmonary disease, define response to treatment, and collect relevant data associated with the diagnosis and treatment of NTM disease to build a framework for true therapeutic trials in the future.
Role: Coordinating Center PI

PROMISE-TDNCC18K1 6/1/2018-5/31/2027 0.3 Calendar Mo.
Cystic Fibrosis Foundation $9,517,693
PROMISE-OB-18 CCC

Prospective study to evaluate biological and clinical effects of significantly corrected CFTR. Objectives for this study are to determine the clinical effectiveness of next generation CFTR modulator drugs, develop a rich specimen biorepository for future research, and enable biological research in a number of related sub studies: microbiology, mucus biology, inflammation, liver disease, endocrinology, gastroenterology, hepatic disease, and imaging studies.
Role: Coordinating Center PI

HAMBLE17K0 (Hamblett) 07/01/23017 – 06/30/2025 1.2 Calendar Mo.
Cystic Fibrosis Foundation $10,078,692
Characterizing CFTR modulated changes in sweat chloride & clinical outcomes
The primary objective is to depict patterns of sweat chloride response across a representative cohort of CF patients receiving CFTR mdulator therapy, adjusting for treatment duration and characterizing these response patterns according to different CFTR modulators and across different patient groups defined by genotype, age, weight, disease severity and disease stage.
Role: Principal Investigator

As Collaborator:

GOSS22Y0 (Goss) 4/1/2023 - 3/31/2025 5.76 Calendar Mo.
Cystic Fibrosis Foundation $11,696,119
Therapeutics Development Network Coordinating Center
The objectives of this coordinating center are to establish and manage a specialized network of research centers throughout the United States to perform clinical studies for the treatment of cystic fibrosis, and to provide the clinical, statistical, and data management infrastructure to support these studies.
Role: Co-Executive Director

SUBMITTED FUNDING

PENDING (Hamblett) 12/01/2024 – 11/30/2031 1.8 Calendar Mo.
National Institutes of Health $16,553,499
Impact of prophylactic azithromycin on outcomes after lung transplant (AZM LTX): A multicenter, randomized placebo-controlled trial – Data Coordinating Center
The objectives are to prevent progressive lung function decline with the use of prophylactic azithromycin to improve quality of life after lung transplant.
Role: Principal Investigator

SELECT PAST FUNDING

SIMPLIFY-HAMBLE20K0 1/1/2020-12/31/2024 0.6 Calender Mo.
Cystic Fibrosis Foundation $7,042,018
Impact of discontinuing chronic therapies in people with CF (SIMPLIFY) DCC
The objectives of this multicenter randomized trial are to determine the impact of withdrawing chronic mucolytic therapy in persons with CF who have initiated highly effective triple combination CFTR modulator therapy.
Role: Principal Investigator

RAMOS22A0-LAD (Ramos) 11/1/2022 – 10/31/2024 0.12 Calendar Mo.

Cystic Fibrosis Foundation $91,589
Planning a multi-center randoized controlled trial of azithromycin to prevent chronic lung allograft dysfunction after lung transplantation
The objectives of this coordinating center are to evaluate feasibility of enrollment, randomization, and continuation of azithromycin, fluticasone/azithromycin/montelukast (FAM), or placebo for 24 months in lung transplant recipients via a survey of clinicians in the Cystic Fibrosis Lung Transplant Consortium (CFLTC); determine rates of prophylactic and therapeutic azithromycin use and incidence of persistent lung function decline and chronic lung allograft dysfunction within 3 years of transplant at CFLTC sites via an electronic health records-based study; assess gastrointestinal (GI) testing practices pre- and post-transplant within the CFLTC to inform clinical trial design.
Role: Co-Investigator

HAMBLE23A0-PG (Hamblett) 03/01/2023 – 5/31/2024 2.4 Calendar Mo.
Cystic Fibrosis Foundation $435,971
Research Expansion to Advance the CF Therapeutics (REACH) Pipeline for People without Modulators Planning Grant
The objectives are to develop programs to reach this patient population to improve communication about research and empower these patients to participate. Specific aims include: 1) Develop a regulatory compliant trial-opportunity communication platform to promote research engagement in pwCF not eligible for HEMT, 2) Conduct a prospective, longitudinal, “light touch” research study to obtain key research quality CF outcome data, 3) Conduct sub-studies to obtain specialized outcome data to inform the evaluation of nucleic acid-based therapies (NABTs) and promote innovative trial designs with the use of shared control data, and 4) Conduct a longitudinal registry based study to characterize the population of people ineligible or intolerant with CF in the CFFPR.
Role: Principal Investigator

1 R01 NR 020470-01A (Ramos) 01/01/2023 – 01/31/2024 0.21 Calendar Mo.
National Institutes of Health
Preparation for lung transplant discussions and decisions among people with cystic fibrosis
The objectives are to (1) Test whether a lung transplant educational resource (Take on Transplant, ToT) improves patient preparedness for discussions about transplant, assessed with the PrepDM Scale, using a multicenter randomized clinical trial; (2) Explore perceptions of use of ToT for lung transplant education through analysis of semi-structured interviews and audio recordings of clinic visits among dyads of people with cystic fibrosis (CF) and their CF physicians; (3) Explore the effect of the ToT intervention on psychosocial functioning.
Role: Co-Investigator

GOAL13K0 (Hamblett) 9/1/2013 - 08/31/2022 0.3 Calendar Mo.
Cystic Fibrosis Foundation $182,488
G551D Observational Study – Expanded to Include Additional Genotypes and Extended for Long Term Follow-up (GOAL e2): Coordinating Center
The primary objective of this study is to obtain specimens for the CFFT Specimen Bank and evaluate clinical and biological outcomes from cystic fibrosis patients with the G551D CFTR mutation, some of whom may be treated with ivacaftor (a novel CFTR potentiator drug).
Role: Prinicipal Investigator

HAMBLE15A0 (Hamblett) 08/01/2015 – 07/31/2022 0.6 Calendar Mo.

Cystic Fibrosis Foundation $200,138
Testing the Effect of Adding Chronic Azithromycin to Inhaled Tobramycin. A randomized, placebo-controlled, double-blinded trial of azithromycin 500mg thrice weekly in combination with inhaled tobramycin: Coordinating Center
To determine if azithromycin impairs the previously-recognized clinical benefits of inhaled tobramycin by comparing changes in pulmonary function as measured by FEV1 between subjects randomized to azithromycin versus placebo during administration of TISP.
Role: Principal Investigator

UL1TR0002319-01 (Disis) 06/01/2017 – 02/28/2022 1.2 Calendar Mo.
NIH $934,825
Institute for Translational Health Sciences-Seattle Children's Subcontract
The objectives of this grant are to support space and infrastructure to conduct translational research studies located at Seattle Children's Hospital. This grant funds the Institute of Translational Health Sciences (ITHS), a partnership between the University of Washington, the Fred Hutchinson Cancer Research Center, Seattle Children's Hospital, and local and regional research and community partners. The ITHS fosters multidisciplinary collaboration, career development, education, innovative technologies, as well as supports the design and conduct of translational health studies.
Role: PhD Biostatistician

5U01HL114623-05 (Ramsey) 9/15/2013 – 6/30/2021 0.9 Calendar Mo.
NIH/NHLBI $2,356,843
Optimizing Treatment for Early Pseudomonas Aeruginosa Infection in Cystic Fibrosis: The OPTIMIZE Multicenter Randomized Trial - CCC
The primary aim of this multicenter randomized trial is: To compare the time to treatment failure, defined as either occurrence of a pulmonary exacerbation or two consecutive quarters of Pa positive cultures, between participants randomized to three times weekly oral placebo and culture-based TIS versus three times weekly oral azithromycin and culture-based TIS.
Role: Co-PI

5R01FD003704-06 (Goss) 07/01/2018 – 06/30/2021 0.6 Calendar Mo.
FDA $321,462
Phase 2 Study of Intravenous Gallium Nitrate in the Treatment of Cystic Fibrosis (IGNITE)
The Seattle Children's team subcontract this project through the UW. This site will perform analyses for the final statistical report, generate the final statistical report, and participate in manuscript preparation for the project.
Role: Principal Investigator

1R01HL124053-03 (Nichols) 08/01/2015 – 03/31/2021 1.2 Calendar Mo.
NIH $600,809
A prospective trial of oral azithromycin when added to inhaled tobramycin in CF: Coordinating Center
This is a double-blinded, randomized, placebo-controlled, crossover withdrawal study of azithromycin in CF subjects who continue cycled, inhaled tobramycin to test the impact of combining azithromycin with inhaled tobramycin therapy.
Role: Co- Investigator

5U01HL114589-05 (Hamblett) 9/15/2013 - 12/30/2020 4.8 Calendar Mo.

NIH/NHLBI $489,598
Optimizing Treatment for Early Pseudomonas Infection in CF Children: Data Coordinating Center
The objectives of this multicenter, randomized study are to evaluate the effectiveness of azithromycin therapy on the treatment of new onset Pseudomonas aeruginosa in children with cystic fibrosis.
Role: Principal Investigator

1UM1HL119073-01 (Goss) 9/1/2013 - 6/30/2018 0.6 Calendar Mo.
NIH $1,653,278
Proof of Principal Evaluation of IV Gallium Nitrate (Ganite™) in Patients with Cystic Fibrosis: Coordinating Center
The objectives of this study are to evaluate the safety and efficacy of IV gallium within the context of a randomized, placebo-controlled clinical trial.
Role: Co-Investigator

SINGH15U0 (Singh) 01/01/2016 – 12/31/2017 0.6 Calendar Mo.
Cystic Fibrosis Foundation Therapeutics $31,718
Pseudomonas biomarkers that predict poor outcomes in cystic fibrosis
The goal of this proposal is to characterize these Pseudomonas aeruginosa isolates in order to enable the correlation of the resulting microbiological characteristics with linked clinical and microbiologic parameters from the source patients.
Role: Co-Investigator

1R21HL129930-01 (Hauser, Hamblett) 09/16/2015 – 06/30/2017 1.2 Calendar Mo.
NIH/NHLBI $32,426
Pseudomonas Genomic Signatures Associated With Persistence in Cystic Fibrosis
The goal of this study is to characterize Pseudomonas aeruginosa isolates in order to enable the correlation of the resulting microbiological characteristics with linked clinical and microbiologic parameters.
Role: PI

5UL1 TR 000423-08 (Disis) 9/17/2007 - 5/31/2017 1.2 Calendar Mo.
NIH $1,124,552
Institute for Translational Health Sciences-Seattle Children's Subcontract
The objectives of this grant are to support space and infrastructure to conduct translational research studies located at Children's Hospital and Regional Medical Center. This grant funds the Institute of Translational Health Sciences (ITHS), a partnership between the University of Washington, the Fred Hutchinson Cancer Research Center, Seattle Children's Hospital, Children's Hospital, and local and regional research and community partners. The ITHS fosters multidisciplinary collaboration, career development, education, innovative technologies, as well as supports the design and conduct of translational health studies.
Role: Director, Seattle Children's Core for Biomedical Statistics

5R01DK095738-02 (Ramsey, Borowitz) 7/15/2012 - 4/30/2017 0.3 Calendar Mo.
NIH/NIDDK $319,582
Baby Observational and Nutritional Study of Cystic Fibrosis (BONUS)
The objectives of this multi-center observational study are to characterize growth in the first year of life among infants with cystic fibrosis, identify clinical actors associated with poor growth, and develop reliable, valid and responsive growth measurements that can be performed at care centers around the country which can be used as efficacy outcomes for future interventional studies in infants with CF.

Role: Biostatistician and Director, Biostatistics and Clinical Data Management

AQUADEK12K0 (Hamblett) 8/1/2012 – 1/31/2017 0.39 Calendar Mo.
Cystic Fibrosis Foundation Therapeutics $507,238
A Multicenter Study of the Effects of AquADEKs-2 on Markers of Inflammation and Oxidative Stress in CF: Coordinating Center
The purpose of this randomized, controlled (Phase II) study will be to further evaluate the effects of a modified formulation of AquADEKs® (AquADEKs-2) on antioxidant levels and markers of inflammation and oxidative stress. The results of this study will be used to determine whether to proceed with a larger Phase III trial targeting a clinical endpoint.
Role: PI

RAMSEY11A0 (Ramsey) 8/1/2011 - 7/31/2016 1.4 Calendar Mo.
Cystic Fibrosis Foundation Therapeutics $237,749
Early Intervention in CF Exacerbation (eICE Study): Coordinating Center
The primary goal of this study is to perform a clinical trial to determine the efficacy of early intervention in the treatment of adolescent and adult CF acute pulmonary exacerbation.
Role: Biostatistician and Director, Biostatistics and Clinical Data Management

CTBM001CUS01T (Hamblett) 12/15/2013 – 12/15/2015 0.6 Calendar Mo.
Novartis $88,204
Long Term Outcomes After Eradication of Pseudomonas Aeruginosa Among Children Enrolled in the EPIC Clinical Trial
The objectives of this study are to characterize long term clinical and microbiologic outcomes among participants of the EPIC clinical trial with cystic fibrosis who received anti-pseudomonal therapy for new onset Pseudomonas aeruginosa.
Role: PI

STARtoo10K1 (Ramsey) 9/1/2010 - 8/31/2015 0.6 Calendar Mo.
Cystic Fibrosis Foundation Therapeutics $213,069
Newly Acquired MRSA Eradication Protocol: Coordinating Center
The objectives of this study are to evaluate the effectivness of an aggressive treatment strategy for methicillin resistant Staphylococcus aureus (MRSA) versus standard of care among individuals with cystic fibrosis infected with MRSA in terms of both microbiologic and clinical outcomes.
Role: Biostatistician and Director, Biostatistics and Clinical Data Management

Pharmaxis, Ltd. (Hamblett) 2/1/2014 – 10/1/2014 0.2 Calendar Mo.
$17,258
Rates of Hemoptysis Among Individuals with Cystic Fibrosis Enrolled in Clinical Trials
The objectives of this study are to estimate rates of hemoptysis among individuals with cystic fibrosis who are receiving standard of care therapy.
Role: PI

R01 HL 098084 (Hamblett/Hoffman) 9/11/2009 - 7/31/2014 1.2 Calendar Mo.
NIH/NHLBI $144,000
Pseudomonas aeruginosa Adaptation During Early CF Airway Infection and Treatment

This translational ancillary study utilizes data and specimens from a completed clinical trial and an ongoing prospective observational study to identify adaptive phenotypic and genotypic changes in Pseudomonas aeruginosa that occur in children with cystic fibrosis, and correlate these changes with clinical outcome.
Role: Co-PI

Gates Foundation (Shaffer) 2/1/2013 – 1/31/2014 0.3 Calendar Mo.
$46,843
Data Management and Statistical Planning for the Hypernatremic Dehydration in Childhood Diarrhea Study
The rationale for this contract is to provide expert oversight in year 1 of the hypernatremia study in the areas of data management and statistical planning. In addition, as part of this collaboration, data management and statistical planning training will be provided for the hypernatremia study research personnel.
Role: Senior Biostatistician

3 P30 DK 47754 (Miller) 1/1/2002 - 12/31/2006 0.6 Calendar Mo.
NIH $753,741
Core Center for Gene Therapy
To provide support for laboratory and clinical pilot projects utilizing gene therapy for the treatment of cystic fibrosis.
Role: Biostatistician

ROSENF03A0 (Rosenfeld) 7/1/2003 – 1/31/2009 0.6 Calendar Mo.
Cystic Fibrosis Foundation Therapeutics $151,871
Evaluation of Expiratory Flows from Raised Volumes as Outcome Measures for Clinical Trials in Infants with Cystic Fibrosis
The objectives of this multicenter observational study are to further the development of a novel procedure to measure pulmonary function in infants with cystic fibrosis and to compare the pulmonary function between infants with cystic fibrosis and healthy controls.
Role: Biostatistician and Director, Biostatistics and Clinical Data Management

1R01DK064954 (Miller) 7/1/2004 – 5/31/2008 1.2 Calendar Mo.
NIH/NHLBI $460,230
Pseudomonas as a Surrogate Maker of Cystic Fibrosis
The objectives of this study are to use existing microbiologic samples to characterize key longitudinal changes in Pseudomonas aeruginosa and correlate these changes with clinical outcome.
Role: Biostatistician

1 U01 HL80310-05 (Ramsey) 9/29/2004 - 3/31/2011 1.2 Calendar Mo.
NIH/NHLBI $1,412,623
Early Antipseudomonal Therapy in Cystic Fibrosis
The objectives of this multicenter, randomized clinical trial are to determine a safe and efficacious approach for the treatment of new onset Pseudomonas aeruginosa in young children with cystic fibrosis.
Role: Biostatistician and Director, Biostatistics and Clinical Data Management

MAC0406K0 (Saiman) 1/1/2006 - 12/31/2009 2.4 Calendar Mo.
Cystic Fibrosis Foundation Therapeutics $2,486,202
Multi-center, Multi-national, Randomized, Placebo-controlled Trial of Azithromycin in participants with Cystic Fibrosis 6-18 years old, Culture Negative for Pseudomonas aeruginosa

The objectives of this randomized trial are to determine the effect of 24 weeks of three times weekly administration of azithromycin as compared to placebo on pulmonary function, safety, and other clinical outcomes among individuals with CF who are culture negative for Pseudomonas aeruginosa.
Role: Biostatistician and Director, Biostatistics and Clinical Data Management

HAMBLE10A0 (Hamblett) 4/1/2010 - 3/31/2011 0.12 Calendar Mo.
Cystic Fibrosis Foundation Therapeutics $127,089
Multicenter Trial to Validate Protein Biomarkers of a Pulmonary Exacerbation in Cystic Fibrosis: Data Coordinating Center
The purpose of this study is to evaluate protein biomarkers measured before and after a pulmonary exacerbation among individuals with cystic fibrosis and to determine a panel of biomarkers associated with clinical response. A second component of this project is to support the development and validation of a patient reported outcome measure that will be submitted to the FDA for approval.
Role: PI

Vertex (Hamblett) 3/15/2011 - 3/14/2012 0.6 Calendar Mo.
Vertex (Investigator Initiated Study) $45,534
Emergence of liver function abnormalities among cystic fibrosis clinical trial participants
The objectives of this study are to further define the frequency and severity of abnormal liver function tests among individuals with cystic fibrosis and to evaluate the association of these abnormalities with potential etiologies and clinical outcomes.
Role: PI

MAC0411KO (Hamblett) 5/1/2011 - 12/31/2012 0.6 Calendar Mo.
Cystic Fibrosis Foundation Therapeutics $36,102
Azithromycin Ancillary Analysis Study
The objectives of this project are to conduct multiple ancillary studies to a multicenter, placebo controlled randomized trial of azithromycin, including an inflammatory marker substudy and studies describing acute antibiotic usage and its association with pulmonary exacerbations.
Role: PI

1 P30 DK089507-01 (Ramsey, Greenberg) 7/6/2010 - 5/31/2015 0.6 Calendar Mo.
NIH/NIDDK $883,752
Translational Research Center to Expedite Novel Therapies in Cystic Fibrosis
The P30 Research Center supports both basic and clinical studies directed towards advancing new therapies to improve and prolong the lives of patients with cystic fibrosis. It supports a large group of researchers at the University of Washington as well as other P30 programs across the US.
Role: Biostatistician and Director, Biostatistics and Clinical Data Management

Novartis Ancillary (Hamblett) 12/16/2011 - 12/16/2012 0.6 Calendar Mo.
Novartis (Investigator Inititated Study) $49,265
Impact of Acute Antibiotic Usage on the Exacerbation Endpoint in CF Clinical Trials
The objectives of this study are to assess the association between acute antibiotic usage for non-pulmonary exacerbation related events and the risk of a pulmonary exacerbation among individuals with cystic fibrosis, and quantify the potential for significant confounding that will bias clinical trial analyses.
Role: PI

BIBLIOGRAPHY

Manuscripts in Peer Reviewed Journals:

1. Mayer-Hamblett N, Self S. A regression modeling approach for describing patterns of HIV genetic variation. *Biometrics*. 2001 Jun;57(2):449-60. PMID: 11414569.
2. Noone PG, Hamblett N, Accurso F, Aitken ML, Boyle M, Dovey M, Gibson R, Johnson C, Kellerman D, Konstan MW, Milgram L, Mundahl J, Retsch-Bogart G, Rodman D, Williams-Warren J, Wilmott RW, Zeitlin P, Ramsey B; Cystic Fibrosis Therapeutics Development Research Group. Safety of aerosolized INS 365 in patients with mild to moderate cystic fibrosis: Results of a phase I multi-center study. *Pediatr Pulmonol*. 2001 Aug;32(2):122-8. PMID: 11477729.
3. Cieri MV, Mayer-Hamblett N, Griffith A, Burns JL. Correlation between *in vitro* and *in vivo* models of invasion in *Burkhoderia cepacia* cystic fibrosis lung infections. *Infect Immun*. 2002 Mar;70(3):1081-6. PMID: 11854186. PMCID: 127769.
4. Goss CH, Hamblett N, Kronmal RA, Ramsey BW. The cystic fibrosis therapeutics development network (CF TDN): a paradigm of a clinical trials network for genetic and orphan diseases. Adv Drug Deliv Rev. 2002;54:1505-28. PMID: 12458158.
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4. Tamara D. Simon*, Kathryn B. Whitlock, Jay Riva-Cambrin, John RW Kestle, Margaret Rosenfeld, J. Michael Dean, Richard Holubkov, Marcie Langley, Nicole Mayer Hamblett. Revision surgeries are associated with significant increased risk of subsequent cerebrospinal fluid shunt infection. Accepted to Pediatric Academic Societies meeting, Boston, Massachusetts for platform presentation April 28, 2012. Presenter Tamara Simon.
5. Quon B S, Psoter KJ, Mayer-Hamblett N, Aitken ML, Li CI, Goss CH. Socioeconomic Status and Access to Lung Transplantation For Adult Cystic Fibrosis Patients In The United States. 2012 North American Cystic Fibrosis Conference. Poster presentation.
6. Hoffman LR, Ramsey BW, Kulasekara HD, Retsch-Bogart GZ, Wolter DJ, Pope CE, Houston LS. Kulasekara BR, Foster JM, Gibson RL, Rosenfeld M, Khan U, Burns JL, Miller S, Mayer-Hamblett N. *Pseudomonas aeruginosa* (*Pa*) Phenotypes Associated with Persistent Early Infection in Cystic Fibrosis (CF) Patients in the Epic Clinical Trial. 2012 North American Cystic Fibrosis Conference. Platform presentation. Presenter Nicole Hamblett.
7. Mayer-Hamblett N, Saiman L, Kloster M, Anstead M, Lands LC, Fisher L, Ratjen F. Impact Of Acute Antibiotic Usage On The Pulmonary Exacerbation Endpoint in a Cystic Fibrosis Randomized Trial. 2012 North American Cystic Fibrosis Conference. Poster presentation.
8. Simon TD*, Butler J, Whitlock KB, Browd S, Holubkov R, Kestle JRW, Kulkarni AW, Langley M, Limbrick DD, Mayer Hamblett N, Tamber M, Wellons III JC, Whitehead WC, Riva-Cambrin C. Cerebrospinal fluid shunt revisions, far more than patient factors, confer risk of shunt infection. Pediatric Academic Societies meeting, Washington, D.C., May 5, 2013. Poster presentation.
9. Hsu E*, Bradford M, Mayer-Hamblett N, Horslen S. Leveling the Playing Field or Gaming the System? Heterogeneity and Disparities in the Use of Exception Scores in Pediatric Liver Allocation. Pediatric Liver Transplantation Conference, Toronto. September 2013. Platform presentation. Presenter Evelyn Hsu.
10. Mayer-Hamblett N, Gibson RL, Rosenfeld M, Ramsey BW, Kulasekara HD, Retsch-Bogart GZ, Wolter DJ, Pope CE, Houston LS, Kulasekara BR, Foster JM, Khan U, Burns JL, Miller SI, Hoffman, LR. *Pseudomonas aeruginosa* Phenotypes Distinguish Initial, Intermittent, And Persistent Stages Of CF Airway Infection. 2013 North American Cystic Fibrosis Conference. 2013, Salt Lake City, UT. Platform presentation. Presenter Nicole Hamblett.

11. Dasenbrook EC, Gemmen E, Sawicki GS, Hamblett NM, Goss CH. Combination Inhaled 7% Hypertonic Saline And Rhdnase Compared To Either Medication Alone Or Neither Therapy: Retrospective Cohort Study of the US CFF National Patient Registry. 2013 North American Cystic Fibrosis Conference. 2013, Salt Lake City, UT. Platform presentation. Presenter E. Dasenbrook.
12. Sanders DB, Fink A, Mayer-Hamblett N, Morgan W, Goss CH. Clinical Events that Precede Pulmonary Exacerbations Predict the Failure to Recover Baseline FEV_1. 2013 North American Cystic Fibrosis Conference. 2013, Salt Lake City, UT. Platform presentation. Presenter DB Sanders.
13. Hsu EK*, Shaffer M, Bradford M, Mayer-Hamblett N, Horslen S. Heterogeneity and Disparities in the Use of Exception Scores in Pediatric Liver Allocation. 2014 World Transplant Congress, San Francisco, CA. Platform presentation. Presenter Evelyn Hsu.
14. Bilton D, Pressler T, Fajac I, Clancy JP, Sands D, Predrag M, Cipolli M, Galeva I, Sole A, Dupont L, Goss CH, Mayer-Hamblett N, Quittner A, Gupta R, Constantine S, Konstan M. Efficacy and safety of once-daily liposomal amikacin for inhalation are comparable with twice-daily tobramycin inhalation solution in cystic fibrosis patients with chronic infection due to *Pseudomonas aeruginosa.* 2014 European Cystic Fibrosis Conference. Platform presentation. Presenter Diana Bilton.
15. Crull MR, Mayer-Hamblett N, Aitken ML, Goss CH. Change in Pseudomonas *aeruginosa* frequency in cystic fibrosis adults over time. 2014 American Thoracic Society Meeting. Poster presentation.
16. Sanders DM, Fink A, Mayer-Hamblett N, Schechter M, Sawicki G, Flume P, Morgan W. Changes in growth parameters predict FEV_1 at age 6 years in young children with cystic fibrosis. 2014 American Thoracic Society Meeting. Poster presentation.
17. Goss CH, West N, Allgood S, Wilhelm E, Khan U, Mayer-Hamblett N, Aitken M L, Ramsey BW, Boyle MP, Mogayzel Jr PJ, Gibson RL, N Lechtzin N for the eICE Study Team. Adherence is high in an ongoing long-term home monitoring trial in CF, the eICE Study. 2014 American Thoracic Society Meeting. Poster presentation.
18. Ramos KJ, Quon BS, Psoter K, Lease ED, Mayer-Hamblett N, Aitken ML, Goss CH. Predictors of late or non-referral for lung transplant evaluation in cystic fibrosis patients with advanced lung disease. 2014 American Thoracic Society Meeting.
19. Hamblett NM, Rosenfeld M, Kloster M, Gibson R, Retsch-Bogart GZ, Thompson V, Ramsey B. Impact of successful eradication of *Pseudomonas aeruginosa* on long term outcomes in cystic fibrosis. 2014 North American Cystic Fibrosis Conference, Atlanta, GA. *Pediatr Pulmonol* Supplement 38; #283:317.
20. Dasenbrook E, Gemmen E, Choodnovskiy I, Sawicki GS, Hamblett NM, Knapp EA, Fink A, Goss CH. Association between the addition of inhaled hypertonic saline to RHDnase and clinical outcomes: propensity score matched analysis of the US CFF Patient Registry. 2014 North American Cystic Fibrosis Conference, Atlanta, GA. *Pediatr Pulmonol* Supplement 38; #380:353.
21. Chmiel J, Hamblett NM, Geller D, Konstan M, VanDevanter D, Thompson V, Molfino N, Yarranton G, Shreeniwas R. A phase2, 16-week, randomized, double-blind, placebo-controlled study to assess safety, tolerability and efficacy of repeated doses of KB001-A in subjects infected with *P aeruginosa.* North American Cystic Fibrosis Conference, Phoenix, AZ. *Pediatr Pulmonol* Supplement 2015; #318:272.
22. Happoldt C, Chmiel J, Hamblett NM, Moskowitz SM, Nick JA, Saiman L, Nichols D. Selective drug interaction between azithromycin and inhaled tobramycin; replication dataset and additional *in vitro* work. North American Cystic Fibrosis Conference, Phoenix, AZ. *Pediatr Pulmonol* Supplement 2015; #292:301.

23. Jorth P, Rezayat A, Hisert KB, Garudathri J, Khan U, Hamblett NM, Singh P. Early evolution of *Pseudomonas aeruginosa* during cystic fibrosis infection. North American Cystic Fibrosis Conference, Phoenix, AZ. *Pediatr Pulmonol* Supplement 2015; #300:304.
24. Crull M, Caldwell E, Hamblett NM, Aitken ML, Goss CH. Change in incidence of chronic *Pseudomonas aeruginosa* in cystic fibrosis adolescents and adults. North American Cystic Fibrosis Conference, Phoenix, AZ. *Pediatr Pulmonol* Supplement 2015; #385:336.
25. Goss CH, West NE, Allgood S, Wilhelm E, Khan U, Howe D, Hamblett NM, Aitken ML, Ramsey BW, Boyle MP, Mogayzel, Jr. PJ, Gibson RL, Orenstein D, Milla C, Lechtzin N. North American Cystic Fibrosis Conference, Phoenix, AZ. *Pediatr Pulmonol* Supplement 2015; Assessment of the effectiveness of home monitoring trial in CF to identify and treat acute pulmonary exacerbation: the eICE study results. North American Cystic Fibrosis Conference, Phoenix, AZ. *Pediatr Pulmonol* Supplement 2015; #402:342.
26. Sanders D, Fink A, Hamblett NM, Flume PA, Morgan WJ, Goss CH. Association between CF Center practices and recovery from pulmonary exacerbation. North American Cystic Fibrosis Conference, Phoenix, AZ. *Pediatr Pulmonol* Supplement 2015; #492:377.
27. Lechtzin N, Allgood S, Kahn U, Hamblett NM, Wilhelm E, West NE, Howe D, Aitken ML, Ramsey BW, Boyle MP, Mogayzel P, Gibson RL, Orenstein D, Milla C, Goss CH, eICE Study Team. The effect of home spirometry and symptom monitoring on treatment adherence in CF. North American Cystic Fibrosis Conference, Phoenix, AZ. *Pediatr Pulmonol* Supplement 2015; #628:421.
28. Muhlebach MS, Thompson V, Popowitch E, Miller MB, Howe D, Baines A, Mayer-Hamblett N, VanDalfsen J, Campbell P, Goss CH. MRSA types in new onset infection – Results from STAR-TOO trial. North American Cystic Fibrosis Conference, Phoenix, AZ. Pediatr Pulmonol Supplement 2015; #360:327.
29. Simon TD, Kronman M, Whitlock K, Gove N, Browd S, Holubkov R, Kulkarni A, Langley M, Limbrick D, Luerssen T, Oakes J, Riva-Cambrin J, Rozzelle C, Shannon C, Tamber M, Wellons J, Whitehead W, Mayer-Hamblett N. Adherence to guidelines in treatment for first cerebrospinal fluid (CSF) shunt infection. EPAS2016: 70: 2165.8.
30. Simon TD, Kronman M, Gove N, Whitlock K, Browd S, Holubkov R, Kestle J, Kulkarni A, Langley M, Limbrick D, Luerssen T, Oakes J, Riva-Cambrin J, Rozzelle C, Shannon C, Tamber M, Wellons J, Whitehead W, Hamblett N. Surgical approach to treatment of first cerebrospinal fluid (CSF) shunt infection is associated with reinfection. EPAS2016: 70: 4450.7.
31. Shaffer M, Mayer-Hamblett N. Efficiency of N-of-1 Studies in Cystic Fibrosis Research. Joint Statistical Meetings. Aug 2016. Platform presentation.
32. Konstan M, McKone E, Moss R, Marigowda G, Cooke J, Lubarsky B, Rubin J, Millar S, Pasta DJ, Hamblett NM, Goss CH, Morgan WJ, Sawicki GS. Evidence Of Reduction In Annual Rate Of FEV_1 Decline And Sustained Benefits With Lumacaftor And Ivacaftor (LUM/IVA) In Patients (PTS) With CF Homozygous For F508DEL-CFTR. 2016 North American Cystic Fibrosis Conference. Platform Presentation.
33. Hamblett NM, VanDevanter DR, Boyle MP. A Study Of The Feasibility Of Placebo-Controlled Trials For The Evaluation Of New CFTR Modulators. 2016 North American Cystic Fibrosis Conference. Poster Presentation.
34. Ramos KJ, Quon BS, Heltshe SL, Hamblett NM, Aitken ML, Goss CH. Heterogeneity In Survival Among Adult Cystic Fibrosis Patients With Low Lung Function. 2016 North American Cystic Fibrosis Conference. Platform Presentation.

35. Shaffer M, Hamblett NM. Potential Of N-Of-1 Studies in Cystic Fibrosis Research. 2016 North American Cystic Fibrosis Conference. Poster Presentation.
36. Sawicki GS, Fink A, Hamblett NM. Uptake Of Lumacaftor-Ivacaftor Use Among Eligible CF Patients In The US In 2015. 2016 North American Cystic Fibrosis Conference. Platform Presentation. Presenter Greg S. Sawicki.
37. Hamblett NM, VanDevanter DR, Rowe SM, Clancy JP, Sagel S, Skalland M, Russell R, Konstan M. Sweat Chloride is Stable Across Decades Among Individuals with CF. North American Cystic Fibrosis Conference, Indianapolis, IN. *Pediatr Pulmonol* 2017; 52(S47):225.
38. Soneji M, Forsberg C, Ozer E, Hamblett NM, Singh P, Hauser AR. Genomic Analysis of *Pseudomonas Aeruginosa* Isolates Causing Early Infection in Cystic Fibrosis. North American Cystic Fibrosis Conference, Indianapolis, IN. *Pediatr Pulmonol* 2017; 52(S47):348.
39. Somayaji R, Muhlebach MS, Ramos KJ, Beckett V, Popowitch E, Miller M, Baines A, Hamblett NM, Zemanick ET, Hoover W, Goss CH. Risk Factors For Methicillin-Resistant *Staphylococcus Aureus* (MRSA) Persistence in Persons with Cystic Fibrosis (CF): Analysis of The STAR-TOO Cohort. North American Cystic Fibrosis Conference, Indianapolis, IN. *Pediatr Pulmonol* 2017; 52(S47):355.
40. Ramsey BW, Retsch-Bogart GZ, Kloster M, Buckingham R, Hamblett NM. Efficacy and Safety of Azithromycin for Treatment of Early *Pseudomonas* in Cystic Fibrosis: The OPTIMIZE Trial. North American Cystic Fibrosis Conference, Indianapolis, IN. *Pediatr Pulmonol* 2017; 52(S47):434.
41. Rowe SM, Khan U, Heltshe S, Donaldson SH, Borowitz D, Gelfond D, Ratjen F, Moran A, Solomon GM, Hamblett NM, Vandalfsen J, Joseloff E, Sagel S, Clancy JP. Results of a Multicenter Prospective Longitudinal Study Evaluating the Effectiveness of Lumacaftor/Ivacaftor in F508Del Homozygous CF Patients Following FDA Approval (PROSPECT Part B Core Study). North American Cystic Fibrosis Conference, Indianapolis, IN. *Pediatr Pulmonol* 2017; 52(S47):437.
42. Somayaji R, Lechtzin N, Ramos KJ, Hamblett NM, West NE, Allgood SJ, Wilhelm E, Khan U, Aitken ML, Ramsey BW, Boyle MP, Mogayzel P, Gibson RL, Orenstein D, Milla C, Clancy JP, Antony V, Goss CH. Lung Function Recovery in Cystic Fibrosis Following a Pulmonary Exacerbation Treated with Intravenous and Oral Antibiotics: Analysis of The EICE Trial. North American Cystic Fibrosis Conference, Indianapolis, IN. *Pediatr Pulmonol* 2017; 52(S47):440.
43. Goss CH, Ostrenga J, Fink A, Flume P, Rosenfeld M, Schechter MS, Hamblett NM, Morgan WJ. Utilizing Cluster Analysis to Identify Clinical Phenotypes in Children with CF. North American Cystic Fibrosis Conference, Indianapolis, IN. *Pediatr Pulmonol* 2017; 52(S47):505.

44. Goss CH, Heltshe S, Aitken ML, Hornick DB, Lechtzin N, McCoy K, Skalland M, Hamblett NM, Wilhelm E, Teresi M, Singh P. The IGNITE Study: IV GALLIUM Nitrate as a Treatment for Chronic *Pseudomonas Aeruginosa* Infection in CF. North American Cystic Fibrosis Conference, Denver, CO. *Pediatr Pulmonol* 2018; 53 (S2); 263.

45. Onchiri FM, Hamblett NM, Gibson R, Morgan WJ, Rosenfeld M, On behalf of EPIC Investigators. Association of Intensity of Antipseudomonal Therapy with Risk of Emergence of Drug-Resistant Pathogens in CF Children with Newly Acquired *Pseudomonas*. North American Cystic Fibrosis Conference, Denver, CO. *Pediatr Pulmonol* 2018; 53 (S2); 281.

46. Soneji M, Ozer E, Kandpal M, Hamblett NM, Singh P, Hauser AR. Using The Accessory Genome of *Pseudomonas Aeruginosa* to Predict Eradication Following New Infection in Cystic Fibrosis. North American Cystic Fibrosis Conference, Denver, CO. *Pediatr Pulmonol* 2018; 53 (S2); 303.
47. Onchiri FM, Goss CH, Ramos KJ, Narkewicz MR, Heltshe S, Mayer-Hamblett N, Aitken ML, Parkins M, Somayaji R. Utility of Noninvasive Tests in the Evaluation of Cystic Fibrosis Liver Disease: A Population-Based Registry Study. North American Cystic Fibrosis Conference, Nashville, TN. Pediatr Pulmonol 2019; 54 (S2): 255.
48. Mayer-Hamblett N, Zemanic ET, Odem-Davis K, VanDevanter DR, Rowe S, Konstan M. CFTR Modulator-Induced Sweat Chloride Changes across the Cystic Fibrosis Population: First Results from the CHEC-SC Study. North American Cystic Fibrosis Conference, Nashville, TN. *Pediatr Pulmonol* 2019; 54 (S2): 229.
49. Paynter A, Goss CH, Heltshe S, Khan U, Lechtzin N, Mayer-Hamblett N. Home Versus Clinic Spirometry to Inform Trial Endpoints in CF: eICE Experience [abstract]. *Pediatric Pulmonology* 2020; 55: S289.
50. N Mayer-Hamblett, JP Clancy,K Odem- Davis,K Pearson, E Zemanick, M Konstan, D VanDevanter on behalf of CHEC-SC Investigators. Clinical Trial Interest after Establishment of Modulator Therapy: Interim CHEC-SC Survey Results. *Journal of CF* 2021; 20 (S2) 555. North American Cystic Fibrosis Conference.
51. M Warden, A Magaret, N Simon, S Heltshe, G Retsch-Bogart, BW Ramsey, N Mayer-Hamblett. A new path for CF clinical trials through the use of historical controls *Journal of CF* 2021; 20 (S2) 47. North American Cystic Fibrosis Conference.
52. Pittman JE, Skalland MS, Sagel SD, Ramsey BW, Mayer-Hamblett N, Retsch-Bogart GZ. Impact of azithromycin on serum inflammatory markers in children with cystic fibrosis and new Pseudomonas *Journal of CF* 2022; S1569-1993(22)00047-9.
53. M. Konstan, N. Mayer-Hamblett, K. Odem-Davis4 , I. Emerman, D. VanDevanter , C. Ren, J. Young , E. Zemanick . Cystic fibrosis transmembrane conductance regulator modulator– induced sweat chloride changes in the cystic fibrosis population from the Characterizing Cystic Fibrosis Transmembrane Conductance Regulator–Modulated Changes in Sweat Chloride Study: 2022 Update, *Journal of Cystic Fibrosis* 21S2 (2022) S1–S378.
54. Guimbellot J, Natt J, Ryan K, Dowell A, Odem-Davis K, Konstan M, Zemanick E, Mayer-Hamblett N, Costa E; Concentrations of elexacaftor/tezacaftor/ivacaftor in the cystic fibrosis population: Interim analysis of the CHEC-Pharmacokinetics study, *Journal of Cystic Fibrosis*, Volume 21, Supplement 2, 2022,Page S154.
55. Mayer-Hamblett N, Fajac I, Konstan M, Zemanick E, Mall M: Greater Reductions in Sweat Chloride With CFTR Modulator Use Are Associated With Improved Clinical Outcomes, *Journal of Cystic Fibrosis (2022,* P694.
56. Warden M, Magaret A, Mooney S, Simon N, Mayer-Hamblett N; Approaches that use historical controls to meet modern needs in cystic fibrosis clinical trials, *Journal of Cystic Fibrosis* 21S2 (2022) S1–S378.
57. VanDevanter DR, Zemanick ET, Konstan MW, Ren CL, Odem-Davis K, Emerman I, Young J, Mayer-Hamblett N; CHEC-SC Study Group. Willingness of people with cystic fibrosis receiving elexacaftor/tezacaftor/ivacaftor (ETI) to participate in randomized modulator and inhaled antimicrobial clinical trials. *Journal of Cystic Fibrosis* 2023 Apr 24:S1569-1993(23)00096-6. doi: 10.1016/j.jcf.2023.04.007. Online ahead of print.
58. Hamblett N, Gifford A, Kloster M, Russell R, Braun A, Gibson R, Hoppe J, Linnemann RW, Liou T, Lysinger J, Milla CE, Young J, Nichols D; Impact of Discontinuing Both Hypertonic Saline and Dornase Alfa in People With CF Established on Elexacaftor/Tezacaftor/Ivacaftor: A SIMPLIFY Ancillary Study. *Journal of Cystic Fibrosis* 2023; 22 (S3): 106.

59. Donaldson S, Corcoran T, Pilewski J, Laube B, Mogayzel P, Ceppe AS, Wu J, Zeman K, Rowe S, Nichols D, Gifford A, Bennett W, Hamblett N; The effect of discontinuing hypertonic saline or dornase alfa on mucociliary clearance in elexacaftor/tezacaftor/ivacaftor treated people with cystic fibrosis: The SIMPLIFY-MCC Study. *Journal of Cystic Fibrosis* 2023; 22 (S3): 113.
60. Rosenfeld M, O'Rourke C, Vu PT, De Fermin Cortes A, Kelly K, Nichols D, Pittman JE, Ratjen F, Solomon GM, Hamblett N, Heltshe SL; 124: Is home spirometry accurate? Comparison of home to office spirometry in the PROMISE study. *Journal of Cystic Fibrosis* 2023; 22 (S3): 124.
61. Polineni D, Cromwell E, Milinic T, Magaret A, Goss CH, Clancy JP; Hamblett N, US Research Participation and Demographics in People with CF who are Ineligible for CFTR Modulators Show an Imminent Need for Trial Engagement Strategies. *Journal of Cystic Fibrosis* 2023; 22 (S3): 136.
62. McElvaney OJ, Milinic T, Robinson K, Pollack L, Hamblett N, Heltshe SL, Cromwell E, Goss CH; 589: The clinical and sociodemographic profile of aging in cystic fibrosis. *Journal of Cystic Fibrosis* 2023; 22 (S3): 589.
63. Sanders DB, Mayer-Hamblett N, Rosenfeld M, Polinieni D, Dasenbrook E, Szczesniak R, Cromwell EA. Characteristics of individuals with cystic fibrosis in the United States ineligible for ivacaftor and elexacaftor/tezacaftor/ivacaftor. *Journal of Cystic Fibrosis.* 2024 Jul 29:S1569-1993(24)00799-9. doi: 10.1016/j.jcf.2024.07.015. Epub ahead of print. PMID: 39079877.
64. Polineni D., Boyarska O., Budhwani H., Clancy J., Donaldson S., Hudson J., Mead A., Pearson K., Ratjen F., Romasco M., VanDalfsen J., Mayer Hamblett N., Research Expansion to Advance the CF Therapeutic Pipeline for People with CF without Modulators, *Journal of Cystic Fibrosis*, Volume 23, Supplement 2, 2024, Page S241, ISSN 1569-1993, https://doi.org/10.1016/S1569-1993(24)01284-0.
65. Basile M., Davis K., Mayer Hamblett N., Riva D., Clancy J., Nichols D., Therapy Initiation and Modification on ETI (TIME): results from CF community engagement focus groups*, Journal of Cystic Fibrosis*, Volume 23, Supplement 2, 2024, Pages S326-S328, ISSN 1569-1993, https://doi.org/10.1016/S1569-1993(24)01432-2.

SELECT INVITED LECTURES AND PRESENTATIONS

1994 "Neural Networks with Applications," Conference on Mathematics, Rose-Hulman University, Terre Haute, IN

2000 "Phase II Study of PFP-3 RSV Vaccine in CF: Primary Outcome, Protection of Lower Respiratory Tract Illness," National Cystic Fibrosis Foundation Meetings, Baltimore, MD

2001 "Improving Estimation of Change in Pulmonary Function in Cystic Fibrosis Clinical Trials," CF Therapeutics Development Network Annual National Meeting, Seattle, WA

2006 "CF Outcome Measures: Moving Drugs through the Pipeline", CF Therapeutics Development Network National Meeting, Seattle, WA

2008 "The Development of Biomarkers for Therapeutic Evaluation – A Statistical Perspective," University of North Carolina Mucociliary and Cough Clearance International Symposium, Raleigh, NC.

2008 "Novel Research Opportunities in CF Clinical Research Utilizing Existing Databases," 2008 American Thoracic Society Meeting, Toronto, ON, Lecture: "From Application to Publication: Statistical Insights, Tips, and Tricks for Secondary Data Analysis."

2008 "Optimal Spirometry Endpoints for Randomized Controlled Trials in Cystic Fibrosis: Percent Predicted or Liters?," North American Cystic Fibrosis Meeting, Orlando, FL.

2009 "Optimizing Outcome Measures for the Next Generation of Cystic Fibrosis Clinical Trials," National Cystic Fibrosis Therapeutics Development Network Meetings, Denver, CO.

2009 "Biomarkers as Tools for Early Disease Detection," NIH Workshop on Biomarkers in CF Liver Disease, Bethesda, MD.

2009 "Safety and Efficacy of Inhaled Tobramycin for Early Eradication of Pseudomonas: The EPIC Study Microbiology Results," North American Cystic Fibrosis Conference, Symposium Session, Minneapolis, MN.

2009 Panel Speaker, Talamo Lecture, Sponsored by the Cystic Fibrosis Foundation and Talamo Endowment, Massachusetts General, Ether Dome.

2010 Instructor and Session Organizer, "Outcomes, Study Design, and Interpretation of CF Clinical Studies," 2010 North American Cystic Fibrosis Conference, Baltimore, MD.

2011 Instructor and Session Organizer, "Current Issues in Statistics for the Cystic Fibrosis Setting", 2011 North American Cystic Fibrosis Conference, Anaheim, CA

2011 "Association between *Pseudomonas aeruginosa* Recurrence and Exacerbations in Children with Cystic Fibrosis: Results from the EPIC Trial." National Cystic Fibrosis Therapeutics Development Network Meetings, Baltimore, MD.

2012 "Understanding and Optimizing the Pulmonary Exacerbation Endpoint: Preparing for the Next Generation of Cystic Fibrosis Clinical Trials," FDA Office of Biostatistics Invited Lecturer, Bethesda, MD

2012 "Statistical analysis of pulmonary exacerbations in clinical trials", FDA Public Workshop, Issues in the Design of Clinical Trials for Antibacterial Drugs for the Treatment of Non-CF Bronchiectasis, Bethesda, MD

2012 "Optimizing the Pulmonary Exacerbation Endpoint: Preparing for the Next Generation of Cystic Fibrosis Clinical Trials", CF Therapeutics Development Network National Meetings, Orlando, FL

2013 Panel Speaker, "Responsible Sharing of Clinical Trial Data", Institute of Medicine, Washington D.C.

2013 "*Pseudomonas aeruginosa* Phenotypes Distinguish Initial, Intermittent, And Persistent Stages Of CF Airway Infection." North American Cystic Fibrosis Foundation Conference, Salt Lake City, UT

2014 Panel Speaker, "Complex Issues in Developing Drug and Biologic Products for Rare Diseases", FDA Public Workshop, Silver Springs, MD

2014 "Facilitating the Development of Second Generation (G2) CFTR Modulators", National Cystic Fibrosis Therapeutics Development Network Meetings, Atlanta, GA

2014 "Impact of successful eradication of Pseudomonas aeruginosa on long term outcomes in cystic fibrosis." North American Cystic Fibrosis Conference, Atlanta, GA

2016 "Next Generation Modulators for Cystic Fibrosis: Developing the Playbook", National Cystic Fibrosis Therapeutics Development Network Meetings, San Antonio, TX

2016 "Sleuthing your Way through the Internet: Valuable Resources to Improve your Understanding of Clinical Trial Results", CF Therapeutics Development Network National Meetings, San Antonio, TX
2017 "Optimizing the Pipeline of CFTR Modulators: Study Design Considerations", CF Canada Broken Arrow Conference, Toronto, ON
2017 "Utilizing External Control Groups in Single Arm Trials", Discussant, FDA-Duke Margolis Think Tank: Exploring Novel Trial Designs and Innovative Statistical Tools in Rare Disease Drug Development, Washington DC
2018 "Efficacy and Safety of Azithromycin for Treatment of Early Pseudomonas Infection in CF: The OPTIMIZE Randomized, Placebo Controlled Trial", CF Research Seminar Series, Seattle Cystic Fibrosis Research and Translation Center (CF-RTC) and the Research Development Program (CF-RDP), University of Washington, Seattle, WA
2018 "Utilizing Patient Registry and Natural History Data to Advance Therapeutic Development for Rare Diseases," Utilizing Innovative Statistical Methods and Trial Designs in Rare Diseases, Duke Margolis/FDA Public Workshop, Washington DC
2018 "Working Towards our Vision: The CF Therapeutics Development Network Coordinating Center Perspective," CF Therapeutics Development Network National Meeting, Philadelphia, PA
2018 "Learning from Clinical Trial Results Beyond Published Data," CF Therapeutics Development Network National Meeting, Philadelphia, PA
2019 "Emerging Issues in the Design of CF Clinical Trials: Hindsight Really is 20/20." CF Data Safety Monitoring Board National Meeting, Phoenix, AZ.
2019 "Let's Talk about Exacerbations!" CF Therapeutics Development Network National Meetings, Austin, TX.
2019 "CFTR Modulator-Induced Sweat Chloride Changes across the Cystic Fibrosis Population: First Results from the CHEC-SC Study. North American Cystic Fibrosis Conference, Nashville, TN.
2019 "Challenging Trial Design Precedent in the Highly Effective Modulator Era." North American Cystic Fibrosis Conference, Nashville, TN.
2020 "Until its Done: Keeping the Therapeutic Pipeline Moving in the Midst of a Breakthrough Therapy in Cystic Fibrosis", National Institute of Allergy and Infectious Diseases (NIAID) Division of Biostatistics, Rockville, MD (virtual)
2020 Panel Speaker, "Data Sharing to Accelerate Therapeutic Development for Rare Diseases," Duke Margolis/FDA Public Workshop, Washington, DC
2020 "Creating a Roadmap for the Development of New CFTR Modulators," European CF Society Clinical Trial Network Meetings, Lyon, France (virtual)
2021 "Study Design Strategy for Small Sample Sizes: From the Practical to the Innovative", Rare Genetic Causes of Bronchiectasis: Paving the Way for Interventional Trials Conference (virtual)
2021 "Clinical Trial Interest after Establishment of Modulator Therapy: Interim CHEC-SC Survey Results," North American Cystic Fibrosis Conference (virtual)
2021 "Clinical Trial Design in the Modulator Era: One Trial does NOT Fit All," North American Cystic Fibrosis Conference (virtual)
2022 "Therapeutic Development and Trial Design in CF: The Next Chapter," Johns Hopkins CF Center for Excellence CF Research Development Program Seminar Series (virtual)
2022 "Current and Future Research Needs in an Era of Highly Effective Modulators," NIH Conference Co-Organizer and Panelist (virtual)
2022 "It Takes Everyone: Clinical Trial Strategy to Advance our Mission," Plenary Speaker at the North American CF Conference, Philadelphia, PN (Audience 7000; 3500 onsite, 3500 online)

2023 "Randomized withdrawal of hypertonic saline in those with lower lung function after receiving elexacaftor/tezacaftor/ivacaftor: A SIMPLIFY Sub-study," European Cystic Fibrosis Society Meetings Vienna, AT
2023 "Advancing the pipeline of cystic fibrosis clinical trials: a new roadmap with a global trial network perspective" European Respiratory Society Meetings, Milan, IT
2023 "Impact of Discontinuing both Hypertonic Saline and Dornase Alfa after Elexacaftor/Tezacaftor/Ivacaftor in Cystic Fibrosis," 2023 North American Cystic Fibrosis Conference, Phoenix, AZ
2024 "An Alternative to Traditional Master Protocols to Advance a Pipeline of Therapies for Cystic Fibrosis", Panelist, National Academy of Science Rare Disease Study, virtual
2024 Enhancing Adoption of Innovative Clinical Trial Approaches, Duke Margolis FDA Workshop, Panelist, Washington DC
2024 "Trial Designs to Progress a Pipeline of Nucleic Acid Based Therapies in Cystic Fibrosis," European CF Clinical Trials Network Meeting, Glasgow, UK
2024 "Clinical Trial Design & Recruitment Considerations to Advance a Pipeline of NABTs," North American Cystic Fibrosis Conference, Boston, MA
2024 "Nucleotide-Based Therapeutics Across Diverse Populations," Session Chair, North American Cystic Fibrosis Conference, Boston, MA
2024 "Finding an Optimal Dose in Drug Development: The Importance of Exposure/Response Relationships for Advancing Chronic CF Therapies," Session Chair, North American Cystic Fibrosis Conference, Boston, MA