CURRICULUM VITAE

Nicole 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

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

<u>Graduate Research Assistant Mentoring:</u>

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-present	Mark Warden, Dept. of Epidemiology, University of Washington

<u>Academic Advisor:</u>

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

<u>Dissertation Committee:</u>

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

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

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
2022-present Omar Bayomy, MD, 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-present DSMB Member, NIAID

Food and Drug Administration:

2014-present Special Government Employee (SGE), Advisory Committee Member
2023 Advisory Committee Member, Pulmonary Division

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-present 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:

MAYFLOWER-HAMBLE21A0-P 1/1/2021-12/312/27 0.3 Calendar Mo.
Cystic Fibrosis Foundation $1,380,812
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: Principal Investigator

HAMBLE22K0 6/1/2022-5/31/2026 0.12 Calendar Mo.
Cystic Fibrosis Foundation
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: Principal Investigator

PROMISE-TDNCC18K1 6/1/2018-5/31/2024 1.8 Calendar Mo.
Cystic Fibrosis Foundation $609,000
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. Enable biological research in a number of related sub studies: microbiology, mucus biology, inflammation, liver disease, endocrinology, gastroenterology, hepatic disease, and imaging studies.
Role: Principal Investigator

SIMPLIFY-HAMBLE20K0 1/1/2020-12/31/2023 2.4 Calender Mo.
Cystic Fibrosis Foundation $283,713
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

HAMBLE17K0 (Hamblett) 07/01/23017 – 06/30/2023 1.2 Calendar Mo.
Cystic Fibrosis Foundation $216,417
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

<u>As Collaborator:</u>

RAMSEY03Y0 (Goss) 4/1/2023 - 3/31/2024 4.28 Calendar Mo.
Cystic Fibrosis Foundation $5,612,374
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

SELECT PAST FUNDING

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.
5. Mayer-Hamblett N, Rosenfeld M, Emerson J, Goss CH, Aitken ML. Developing cystic fibrosis lung transplant referral criteria using predictors of two year mortality. *Am J Respir Crit Care Med*. 2002 Dec 15;166(12 Pt 1):1550-5. PMID: 12406843.
6. Gibson RL, Emerson J, McNamara S, Burns JL, Rosenfeld M, Yunker A, Hamblett N, Accurso F, Dovey M, Hiatt P, Konstan MW, Moss R, Retsch-Bogart G, Wagener J, Waltz D, Wilmott R, Zeitlin PL, Ramsey B; Cystic Fibrosis Therapeutics Development Network Study Group. Significant microbiological effect of inhaled tobramycin in young children with cystic fibrosis. *Am J Respir Crit Care Med*. 2003 Mar 15;167(6):841-9. PMID: 12480612.
7. Smith AL, Fiel SB, Mayer-Hamblett N, Ramsey B, Burns JL. Susceptibility testing of *Pseudomonas aeruginosa* isolates and clinical response to parenteral antibiotic administration: lack of association in cystic fibrosis. *Chest.* 2003 May;123(5):1495-502. PMID: 12740266.
8. Piedra PA, Cron SG, Jewell A, Hamblett N, McBride R, Palacio MA, Ginsberg R, Oermann CM, Hiatt PW; Purified Fusion Protein Vaccine Study Group. Immunogenicity of a new purified fusion protein vaccine to respiratory synctial virus: A multi-center, placebo-controlled trial in children with cystic fibrosis. *Vaccine*. 2003 Jun 2;21(19-20):2448-60. PMID: 12744878.
9. Ordonez CL, Henig NR, Mayer-Hamblett N, Accurso FJ, Burns JL, Chmiel JF, Daines CL, Gibson RL, McNamara S, Retsch-Bogart GZ, Zeitlin PL, Aitken ML. Inflammatory and Microbiologic Markers in Induced Sputum Following IV Antibiotics in Cystic Fibrosis. *Am J Respir Crit Care Med*. 2003 Dec 15;168(12):1471-5. PMID: 12969869.
10. Saiman L[†], Marshall BC[†], Mayer-Hamblett N, Burns JL, Quittner AL, Cibene DA, Coquillette S, Fieberg AY, Accurso FJ, Campbell PW 3rd; Macrolide Study Group. [†]Co-Principal Investigators and first authors. A multi-center, randomized, placebo controlled, double-blind trial of azithromycin in patients with cystic fibrosis chronically infected with *Pseudomonas aeruginosa*. *JAMA.* 2003 Oct 1;290(13):1749-56. PMID: 14519709.

11. Moss RB, Rodman D, Spencer LT, Aitken ML, Zeitlin PL, Waltz D, Milla C, Brody AS, Clancy JP, Ramsey B, Hamblett N, Heald AE. Repeated adeno-associated virus serotype 2 aerosol-mediated cystic fibrosis transmembrane regulator gene transfer to the lungs of patients with cystic fibrosis: a multicenter, double-blind, placebo-controlled trial. *Chest.* 2004 Feb;125(2):509-21. PMID: 14769732.

12. Goss CH, Mayer-Hamblett N, Aitken ML, Rubenfeld GD, Ramsey BW. Association between *Stenotrophomonas maltophilia* and lung function in Cystic Fibrosis. *Thorax.* 2004 Nov;59(11):955-9. PMID: 15516471. PMCID: 1746887.

13. Beckmann C, Brittnacher M, Ernst R, Mayer-Hamblett N, Miller SI, Burns JL. Use of phage display to identify potential Pseudomonas aeruginosa gene products relevant to early cystic fibrosis airway infections. *Infect Immun.* 2005 Jan;73(1):444-52. PMID: 15618183. PMCIDl 538986.

14. Moss RB, Mayer-Hamblett N, Wagener J, Daines C, Hale K, Ahrens R, Gibson RL, Anderson P, Retsch-Bogart G, Nasr SZ, Noth I, Waltz D, Zeitlin P, Ramsey B, Starko K. A Randomized, Double-Blind, Placebo-Controlled, Dose-Escalating Study of Aerosolized Interferon γ-1b in Patients with Mild to Moderate Cystic Fibrosis Lung Disease. *Pediatr Pulmonol*. 2005 Mar;39(3):209-18. PMID: 15573395.

15. Mayer-Hamblett N, Kronmal RA. Improving the estimation of change from baseline in a continuous outcome measure in the clinical trial setting. *Contemp Clin Trials.* 2005 Feb;26(1):2-16. PMID: 15837448.

16. Saiman L, Mayer-Hamblett N, Campbell P, Marshall BC; Macrolide Study Group. Heterogeneity of Treatment Response to Azithromycin in Patients with CF Chronically Infected with *P. aeruginosa*. *Am J Respir Crit Care Med.* 2005 Oct 15;172(8):1008-12. PMID: 16040785.

17. Goss CH, Mayer-Hamblett N, Kronmal RA, Williams J, Ramsey BW. Laboratory Parameter Profiles among Patients with Cystic Fibrosis. *J Cyst Fibros*. 2007 Apr;6(2):117-23. PMID: 16829217.

18. Mayer-Hamblett N, Aitken ML, Accurso FJ, Kronmal RA, Konstan MW, Burns JL, Sagel SD, Ramsey BW. Association Between Pulmonary Function and Sputum Biomarkers in Cystic Fibrosis. *Am J Respir Crit Care Med*. 2007 Apr 15;175(8):822-8. PMID: 17234902. PMCID: 2720115.

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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. J. Guimbellot, J. Natt, K. Ryan, A. Dowell, K. Odem-Davis, M. Konstan, E. Zemanick, N. Mayer-Hamblett, E. Acosta, 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. Nicole Mayer-Hamblett, Isabelle Fajac, Michael Konstan, Edith Zemanick, Marcus Mall, Greater Reductions in Sweat Chloride With CFTR Modulator Use Are Associated With Improved Clinical Outcomes, *Journal of Cystic Fibrosis (20220,* P694.
56. M. Warden, A. Magaret, S. Mooney, N. Simon, N. Mayer-Hamblett, Approaches that use historical controls to meet modern needs in cystic fibrosis clinical trials, *Journal of Cysitc 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.

SELECT INVITED LECTURES AND PRESENTATIONS

Regional

1999 "A Regression Modeling Approach for Describing Patterns of HIV Genetic Variation," University of Washington Department of Biostatistics Seminar, University of Washington, Seattle, WA

2005 "Improving the Estimation of Change in the Clinical Trial Setting", Childrens Health Institute, Department of Pediatrics, University of Washington, Seattle, WA GA

2015 "Optimizing Treatment for Early *Pseudomonas* Infections in Cystic Fibrosis – The OPTIMIZE Trial: A Data Coordinating Center (DCC) Perspective", CCTR Science Day, Seattle, WA.

National

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