

Last Updated July 2023

Original Research:

Wainwright C, McColley SA, McNally P, Powers M, Ratjen F, Rayment JH, **Retsch-Bogart G**, Roesch E, Ahluwalia N, Chin A, Chu C, Lu M, Menon P, Waltz D, Weinstock T, Zelazoski L, Davies JC. Long-Term Safety and Efficacy of Elexacaftor/Tezacaftor/Ivacaftor in Children Aged ≥ 6 Years with Cystic Fibrosis and at Least One F508del Allele: A Phase 3, Open-Label Clinical Trial. *Am J Respir Crit Care Med*. 2023 Jul 1;208(1):68-78. doi: 10.1164/rccm.202301-0021OC. PMID: 37154609

Mayer-Hamblett N, Ratjen F, Russell R, Donaldson SH, Riekert KA, Sawicki GS, Odem-Davis K, Young JK, Rosenbluth D, Taylor-Cousar JL, Goss CH, **Retsch-Bogart G**, Clancy JP, Genatossio A, O'Sullivan BP, Berlinski A, Millard SL, Omlor G, Wyatt CA, Moffett K, Nichols DP, Gifford AH; SIMPLIFY Study Group. Discontinuation versus continuation of hypertonic saline or dornase alfa in modulator treated people with cystic fibrosis (SIMPLIFY): results from two parallel, multicentre, open-label, randomised, controlled, non-inferiority trials. *Lancet Respir Med*. 2023 Apr;11(4):329-340. doi: 10.1016/S2213-2600(22)00434-9. Epub 2022 Nov 4. PMID: 36343646 Clinical Trial.

Tiddens HAWM, Chen Y, Andrinopoulou ER, Davis SD, Rosenfeld M, Ratjen F, Kronmal RA, Hinckley Stukovsky KD, Dasiewicz A, Stick SM; SHIP-CT Study Group. The effect of inhaled hypertonic saline on lung structure in children aged 3-6 years with cystic fibrosis (SHIP-CT): a multicentre, randomised, double-blind, controlled trial. *Lancet Respir Med*. 2022 Jul;10(7):669-678. doi: 10.1016/S2213-2600(21)00546-4. Epub 2022 Mar 11. PMID: 35286860 Clinical Trial.

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*. *J Cyst Fibros*. 2022 Mar 5:S1569-1993(22)00047-9. doi: 10.1016/j.jcf.2022.02.015. Online ahead of print. PMID: 35260354

Magaret AS, Warden M, Simon N, Heltshe S, **Retsch-Bogart GZ**, Ramsey BW, Mayer-Hamblett N. A new path for CF clinical trials through the use of historical controls. *J Cyst Fibros*. 2022 Mar;21(2):293-299. doi: 10.1016/j.jcf.2021.11.007. Epub 2021 Dec 5. PMID: 34879997

Nichols DP, Singh PK, Baines A, Caverly LJ, Chmiel JF, Gibson RL, Lascano J, Morgan SJ, **Retsch-Bogart G**, Saiman L, Sadeghi H, Billings JL, Heltshe SL, Kirby S, Kong A, Nick JA, Mayer-Hamblett N; TEACH Study Group. Testing the effects of combining azithromycin with inhaled tobramycin for *P. aeruginosa* in cystic fibrosis: a randomised, controlled clinical trial. *Thorax*. 2021 Oct 27;thoraxjnl-2021-217782. doi: 10.1136/thoraxjnl-2021-217782. Online ahead of print. PMID: 34706982

Stanojevic S, Davis SD, Perrem L, Shaw M, **Retsch-Bogart G**, Davis M, Jensen R, Clem CC, Isaac SM, Guido J, Jara S, France L, McDonald N, Solomon M, Sweezey N, Grasemann H, Waters V, Sanders DB, Ratjen FA. Determinants of lung disease progression measured by lung clearance index in children with cystic fibrosis. *Eur Respir J*. 2021 Feb 4:2003380. doi: 10.1183/13993003.03380-2020. Online ahead of print. PMID: 33542049

Mayer-Hamblett N, Nichols DP, Odem-Davis K, Riekert KA, Sawicki GS, Donaldson SH, Ratjen F, Konstan MW, Simon N, Rosenbluth DB, **Retsch-Bogart G**, Clancy JP, VanDalfsen JM, Buckingham R, Gifford AH; Cystic Fibrosis Therapeutics Development Network and SIMPLIFY Investigators. Evaluating the Impact of Stopping Chronic Therapies after Modulator Drug Therapy in Cystic Fibrosis: The SIMPLIFY Study Design. *Ann Am Thorac Soc*. 2021 Jan 19. doi: 10.1513/AnnalsATS.202010-1336SD. Online ahead of print. PMID: 33465316

Pearson K, Mayer-Hamblett N, Goss CH, **Retsch-Bogart GZ**, VanDalfsen JM, Burks P, Rosenbluth D, Clancy JP, Hoffman A, Nichols DP; The impact of SARS-CoV-2 on the cystic fibrosis foundation therapeutics development

network. *J Cyst Fibros.* 2021 Mar;20(2):195-197. doi: 10.1016/j.jcf.2020.12.007. Epub 2020 Dec 15. PMID: 33349583

Mayer-Hamblett N, van Koningsbruggen-Rietschel S, Nichols DP, VanDevanter DR, Davies JC, Lee T, Durmowicz AG, Ratjen F, Konstan MW, Pearson K, Bell SC, Clancy JP, Taylor-Cousar JL, De Boeck K, Donaldson SH, Downey DG, Flume PA, Drevinek P, Goss CH, Fajac I, Magaret AS, Quon BS, Singleton SM, VanDalfsen JM, **Retsch-Bogart GZ**. Building global development strategies for CF therapeutics during a transitional CFTR modulator era. *J Cyst Fibros.* 2020 Jun 7:S1569-1993(20)30161-2. doi: 10.1016/j.jcf.2020.05.011. Online ahead of print. PMID: 32522463

Perrem L, Stanojevic S, Shaw M, Davis S, **Retsch-Bogart G**, Ratjen F. Changes in the parent cystic fibrosis questionnaire-revised (CFQ-R) with respiratory symptoms in preschool children with cystic fibrosis. *J Cyst Fibros.* 2020 Mar 2:S1569-1993(20)30066-7. doi: 10.1016/j.jcf.2020.02.017. Online ahead of print. PMID: 32139196

Shaw M, Oppelaar MC, Jensen R, Stanojevic S, Davis SD, **Retsch-Bogart G**, Ratjen FA. The utility of moment ratios and abbreviated endpoints of the multiple breath washout test in preschool children with cystic fibrosis. *Pediatr Pulmonol.* 2020 Mar;55(3):649-653. doi: 10.1002/ppul.24618. Epub 2020 Jan 3. PMID: 31899855

Mayer-Hamblett N, **Retsch-Bogart G**, Kloster M, Accurso F, Rosenfeld M, Albers G, Black P, Brown P, Cairns A, Davis SD, Graff GR, Kerby GS, Orenstein D, Buckingham R, Ramsey BW; OPTIMIZE Study Group. Azithromycin for Early Pseudomonas Infection in Cystic Fibrosis. The OPTIMIZE Randomized Trial. *Am J Respir Crit Care Med.* 2018 Nov 1;198(9):1177-1187. doi: 10.1164/rccm.201802-0215OC. PMID:29890086

Rayment JH, Stanojevic S, Davis SD, **Retsch-Bogart G**, Ratjen F. Lung clearance index to monitor treatment response in pulmonary exacerbations in preschool children with cystic fibrosis. *Thorax.* 2018 Feb 15. pii: thoraxjnl-2017-210979. doi: 10.1136/thoraxjnl-2017-210979. [Epub ahead of print] PMID: 29449440

Zemanick ET, Wagner BD, Robertson CE, Ahrens RC, Chmiel JF, Clancy JP, Gibson RL, Harris WT, Kurland G, Laguna TA, McColley SA, McCoy K, **Retsch-Bogart G**, Sobush KT, Zeitlin PL, Stevens MJ, Accurso FJ, Sagel SD, Harris JK. Airway microbiota across age and disease spectrum in cystic fibrosis. *Eur Respir J.* 2017 Nov 16;50(5). pii: 1700832. doi: 10.1183/13993003.00832-2017. Print 2017 Nov. PMID: 29146601

Oude Engberink E, Ratjen F, Davis SD, **Retsch-Bogart G**, Amin R, Stanojevic S. Inter-test reproducibility of the lung clearance index measured by multiple breath washout. *Eur Respir J.* 2017 Oct 5;50(4). pii: 1700433. doi: 10.1183/13993003.00433-2017. Print 2017 Oct. PMID: 28982773

Stanojevic S, Davis SD, **Retsch-Bogart G**, Webster H, Davis M, Johnson RC, Jensen R, Pizarro ME, Kane M, Clem CC, Schornick L, Subbarao P, Ratjen FA. Progression of Lung Disease in Preschool Patients with Cystic Fibrosis. *Am J Respir Crit Care Med.* 2017 May 1;195(9):1216-1225. doi: 10.1164/rccm.201610-2158OC. PMID: 27943680

Flume PA, Clancy JP, **Retsch-Bogart GZ**, Tullis DE, Bresnik M, Derchak PA, Lewis SA, Ramsey BW. Continuous alternating inhaled antibiotics for chronic pseudomonal infection in cystic fibrosis. *J Cyst Fibros.* 2016 May 24. pii: S1569-1993(16)30050-9. doi: 10.1016/j.jcf.2016.05.001. [Epub ahead of print] PMID: 27233377

Abode KA, Drake AF, Zdanski CJ, **Retsch-Bogart GZ**, Gee AB, Noah TL. A Multidisciplinary Children's Airway Center: Impact on the Care of Patients With Tracheostomy. *Pediatrics.* 2016 Feb;137(2):e20150455. doi: 10.1542/peds.2015-0455. Epub 2016 Jan 11. PMID: 26755695

Mayer-Hamblett N, Kloster M, Rosenfeld M, Gibson RL, **Retsch-Bogart GZ**, Emerson J, Thompson V, Ramsey BW. Impact of Sustained Eradication of New Pseudomonas aeruginosa Infection on Long Term Outcomes in Cystic Fibrosis. *Clin Infect Dis.* 2015 May 13. pii: civ377. [Epub ahead of print] PMID: 25972024

Retsch-Bogart GZ, Van Dalfsen JM, Marshall BC, George C, Pilewski JM, Nelson EC, Goss CH, Ramsey BW. Highly effective cystic fibrosis clinical research teams: critical success factors. *J Gen Intern Med.* 2014 Aug;29 Suppl 3:714-23. doi: 10.1007/s11606-014-2896-8.

Mayer-Hamblett N, Rosenfeld M, Gibson RL, Ramsey BW, Kulasekara HD, **Retsch-Bogart GZ**, Morgan W, Wolter DJ, Pope CE, Houston LS, Kulasekara BR, Khan U, Burns JL, Miller SI, Hoffman LR. *Pseudomonas aeruginosa* in vitro phenotypes distinguish cystic fibrosis infection stages and outcomes. *Am J Respir Crit Care Med.* 2014 Jun 17. [Epub ahead of print] PMID: 24937177

Mayer-Hamblett N, Ramsey BW, Kulasekara H, Wolter DJ, Houston L, Pope C, Kulasekara B, Armbruster C, Burns JL, **Retsch-Bogart G**, Rosenfeld M, Gibson RL, Miller SI, Khan U, Hoffman LR. *Pseudomonas aeruginosa* Phenotypes Associated with Eradication Failure in Children with Cystic Fibrosis. *Clin Infect Dis.* 2014 May 26. pii: ciu385. [Epub ahead of print] PMID:24863401

Calloway HE, Kimbell JS, Davis SD, **Retsch-Bogart GZ**, Pitkin EA, Abode K, Superfine R, Zdanski CJ. Comparison of endoscopic versus 3D CT derived airway measurements. *Laryngoscope.* 2013 Sep;123(9):2136-41

Tullis E, Burns JL, **Retsch-Bogart GZ**, Bresnik M, Henig NR, Lewis SA, LiPuma JL. Inhaled aztreonam for chronic *Burkholderia* infection in cystic fibrosis: a placebo-controlled trial. *J Cyst Fibros.* 2014 May;13(3):296-305. doi: 10.1016/j.jcf.2013.08.011. Epub 2013 Oct 28. *J Cyst Fibr.* 2013 [Epub ahead of print]

Mayer-Hamblett N, Rosenfeld M, Treggiari MM, Konstan MW, **Retsch-Bogart G**, Morgan W, Wagener J, Gibson RL, Khan U, Emerson J, Thompson V, Elkin EP, Ramsey BW; for the EPIC, ESCF Investigators. Standard care versus protocol based therapy for new onset *Pseudomonas aeruginosa* in cystic fibrosis. *Pediatr Pulmonol.* 2013 Jul 2. doi: 10.1002/ppul.22693. [Epub ahead of print]

Mayer-Hamblett N, Kronmal RA, Gibson RL, Rosenfeld M, **Retsch-Bogart G**, Treggiari MM, Burns JL, Khan U, Ramsey BW; EPIC Investigators. Initial *Pseudomonas aeruginosa* treatment failure is associated with exacerbations in cystic fibrosis. *Pediatr Pulmonol.* 2012 Feb;47(2):125-34. doi: 10.1002/ppul.21525. Epub 2011 Aug 9.

Oermann CM, McCoy KS, **Retsch-Bogart GZ**, Gibson RL, McKeivitt M, Montgomery AB. *Pseudomonas aeruginosa* antibiotic susceptibility during long-term use of aztreonam for inhalation solution (AZLI). *J Antimicrob Chemother.* 2011 Oct;66(10):2398-404. Epub 2011 Jul 22. PMID:21784781

Treggiari MM, **Retsch-Bogart G**, Mayer-Hamblett N, Khan U, Kulich M, Kronmal R, Williams J, Hiatt P, Gibson RL, Spencer T, Orenstein D, Chatfield BA, Froh DK, Burns JL, Rosenfeld M, Ramsey BW; Early *Pseudomonas* Infection Control (EPIC) Investigators. Comparative efficacy and safety of 4 randomized regimens to treat early *Pseudomonas aeruginosa* infection in children with cystic fibrosis. *Arch Pediatr Adolesc Med.* 2011 Sep;165(9):847-56.

Oermann CM, **Retsch-Bogart, GZ**, Quittner AL, Gibson RL, McCoy KS, Montgomery AB, Cooper PJ. An 18-month study of the safety and efficacy of repeated courses of inhaled aztreonam lysine in cystic fibrosis. *Pediatr Pulmonol.* 2010 Nov;45(11):1121-34. PubMed PMID: 20672296.

Rosenfeld M, Emerson J, McNamara S, Joubran K, **Retsch-Bogart, G**, Graff GR, Gutierrez HH, Kanga JF, Lahiri T, Noyes B, Ramsey B, Ren CL, Schechter M, Morgan W, Gibson RL; EPIC Study Group Participating Clinical Sites. Baseline characteristics and factors associated with nutritional and pulmonary status at enrollment in the cystic fibrosis EPIC observational cohort. *Pediatr Pulmonol.* 2010 Sep;45(9):934-44. PubMed PMID: 20597081.

Retsch-Bogart, GZ, Quittner AL, Gibson RL, Oermann CM, McCoy KS, Montgomery AB, Cooper PJ. Efficacy and safety of inhaled aztreonam lysine for airway pseudomonas in cystic fibrosis. *Chest*. 2009 May;135(5):1223-32. PubMed PMID: 19420195; PubMed Central PMCID: PMC2818415.

Treggiari MM, Rosenfeld M, Mayer-Hamblett N, **Retsch-Bogart, G**, Gibson RL, Williams J, Emerson J, Kronmal RA, Ramsey BW; EPIC Study Group. Early anti-pseudomonal acquisition in young patients with cystic fibrosis: rationale and design of the EPIC clinical trial and observational study'. *Contemp Clin Trials*. 2009 May;30(3):256-68. Epub 2009 Jan 15. PubMed PMID: 19470318; PubMed Central PMCID: PMC2783320.

McCoy KS, Quittner AL, Oermann CM, Gibson RL, **Retsch-Bogart, GZ**, Montgomery AB. Inhaled aztreonam lysine for chronic airway Pseudomonas aeruginosa in cystic fibrosis. *Am J Respir Crit Care Med*. 2008 Nov 1;178(9):921-8. Epub 2008 Jul 24. PubMed PMID: 18658109; PubMed Central PMCID: PMC2577727.

Retsch-Bogart, GZ, Burns JL, Otto KL, Liou TG, McCoy K, Oermann C, Gibson RL; AZLI Phase II Study Group. A phase 2 study of aztreonam lysine for inhalation to treat patients with cystic fibrosis and Pseudomonas aeruginosa infection. *Pediatr Pulmonol*. 2008 Jan;43(1):47-58. PubMed PMID: 18041081.

Treggiari MM, Rosenfeld M, **Retsch-Bogart, G**, Gibson R, Ramsey B. Approach to eradication of initial Pseudomonas aeruginosa infection in children with cystic fibrosis. *Pediatr Pulmonol*. 2007 Sep;42(9):751-6. PubMed PMID: 17647287.

Gibson RL, Emerson J, Mayer-Hamblett N, Burns JL, McNamara S, Accurso FJ, Konstan MW, Chatfield BA, **Retsch-Bogart, G**, Waltz DA, Acton J, Zeitlin P, Hiatt P, Moss R, Williams J, Ramsey BW. Duration of treatment effect after tobramycin solution for inhalation in young children with cystic fibrosis. *Pediatr Pulmonol*. 2007 Jul;42(7):610-23. PubMed PMID: 17534969.

Davis SD, Fordham LA, Brody AS, Noah TL, **Retsch-Bogart, GZ**, Qaqish BF, Yankaskas BC, Johnson RC, Leigh MW. Computed tomography reflects lower airway inflammation and tracks changes in early cystic fibrosis. *Am J Respir Crit Care Med*. 2007 May 1;175(9):943-50. Epub 2007 Feb 15. PubMed PMID: 17303797.

Gibson RL, **Retsch-Bogart, GZ**, Oermann C, Milla C, Pilewski J, Daines C, Ahrens R, Leon K, Cohen M, McNamara S, Callahan TL, Markus R, Burns JL. Microbiology, safety, and pharmacokinetics of aztreonam lysinate for inhalation in patients with cystic fibrosis. *Pediatr Pulmonol*. 2006 Jul;41(7):656-65. PubMed PMID: 16703579.

Deterding R, **Retsch-Bogart, G**, Milgram L, Gibson R, Daines C, Zeitlin PL, Milla C, Marshall B, Lavange L, Engels J, Mathews D, Gorden J, Schaberg A, Williams J, Ramsey B; Cystic Fibrosis Foundation Therapeutics Development Network. Safety and tolerability of denufosal tetrasodium inhalation solution, a novel P2Y2 receptor agonist: results of a phase 1/phase 2 multicenter study in mild to moderate cystic fibrosis. *Pediatr Pulmonol*. 2005 Apr;39(4):339-48. PubMed PMID: 15704203.

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. Randomized, double-blind, placebo-controlled, dose-escalating study of aerosolized interferon gamma-1b in patients with mild to moderate cystic fibrosis lung disease. *Pediatr Pulmonol*. 2005 Mar;39(3):209-18. PubMed PMID: 15573395.

Ordoñez 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 after intravenous antibiotics in cystic fibrosis. *Am J Respir Crit Care Med*. 2003 Dec 15;168(12):1471-5. Epub 2003 Sep 11. PubMed PMID: 12969869.

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. Epub 2002 Dec 12. PubMed PMID: 12480612.

Egan TM, Detterbeck FC, Mill MR, Bleiweis MS, Aris R, Paradowski L, **Retsch-Bogart, G**, Mueller BS. Long term results of lung transplantation for cystic fibrosis. *Eur J Cardiothorac Surg*. 2002 Oct;22(4):602-9. PubMed PMID: 12297180.

Rosenfeld M, Gibson R, McNamara S, Emerson J, McCoyd KS, Shell R, Borowitz D, Konstan MW, **Retsch-Bogart, G**, Wilmott RW, Burns JL, Vicini P, Montgomery AB, Ramsey B. Serum and lower respiratory tract drug concentrations after tobramycin inhalation in young children with cystic fibrosis. *J Pediatr*. 2001 Oct;139(4):572-7. PubMed PMID: 11598606.

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. PubMed PMID: 11477729.

Scott CS, **Retsch-Bogart, GZ**, Henry MM. Renal failure and vestibular toxicity in an adolescent with cystic fibrosis receiving gentamicin and standard-dose ibuprofen. *Pediatr Pulmonol*. 2001 Apr;31(4):314-6. PubMed PMID: 11288217.

Dong JQ, Ni L, Scott CS, **Retsch-Bogart, GZ**, Smith PC. Pharmacokinetics of ibuprofen enantiomers in children with cystic fibrosis. *J Clin Pharmacol*. 2000 Aug;40(8):861-8. PubMed PMID: 10934670.

Scott CS, **Retsch-Bogart, GZ**, Kustra RP, Graham KM, Glasscock BJ, Smith PC. The pharmacokinetics of ibuprofen suspension, chewable tablets, and tablets in children with cystic fibrosis. *J Pediatr*. 1999 Jan;134(1):58-63. PubMed PMID: 9880450.

LeGrys VA, **Retsch-Bogart, GZ**. Urticaria associated with the pilocarpine iontophoresis sweat test. *Pediatr Pulmonol*. 1997 Oct;24(4):296-7. PubMed PMID: 9368265.

Retsch-Bogart, GZ, Moats-Staats BM, Howard K, D'Ercole AJ, Stiles AD. Cellular localization of messenger RNAs for insulin-like growth factors (IGFs), their receptors and binding proteins during fetal rat lung development. *Am J Respir Cell Mol Biol*. 1996 Jan;14(1):61-9. PubMed PMID: 8534487.

Moats-Staats BM, **Retsch-Bogart, GZ**, Price WA, Jarvis HW, D'Ercole AJ, Stiles AD. Insulin-like growth factor-I (IGF-I) antisense oligodeoxynucleotide mediated inhibition of DNA synthesis by WI-38 cells: evidence for autocrine actions of IGF-I. *Mol Endocrinol*. 1993 Feb;7(2):171-80. PubMed PMID: 7682287.

Retsch-Bogart, GZ, Stiles AD, Moats-Staats BM, Van Scott MR, Boucher RC, D'Ercole AJ. Canine tracheal epithelial cells express the type 1 insulin-like growth factor receptor and proliferate in response to insulin-like growth factor I. *Am J Respir Cell Mol Biol*. 1990 Sep;3(3):227-34. PubMed PMID: 2167699.