Pediatrics 2017 Annual Report – Division Highlights

Pulmonology & Sleep Medicine

DIVISION DESCRIPTION

The Division of Pediatric Pulmonology and Sleep Medicine provides interdisciplinary family-centered care for children with respiratory disorders, including asthma, cystic fibrosis, sleep disorders, neuromuscular disorders, and chronic lung disease.

The division continues to build upon leading-edge clinical research into the early diagnosis and epidemiology of cystic fibrosis with new investigations into the disease's microbiome. Additional clinical research focuses on environment-disease interactions in asthma, pulmonary management of children with neuromuscular disorders, education and effect of sleep disorders in children. The division also has an active translational research program that is investigating the role of stem and progenitor cells in newborn lung disease. Finally, we host one of two pediatric sleep medicine programs in Wisconsin.

2017 HIGHLIGHTS

- Division Chief **Vivek Balasubramaniam, MD**, received the 2017 Thomas A. Hazinski Distinguished Service Award from the Society for Pediatric Research (SPR) (link to YIR story on faculty awards). Dr. Balasubramaniam was selected in recognition of his multiple contributions to SPR, including co-chairing its advocacy committee and presenting as part of SPR's first congressional briefing in Washington, DC, on the critical importance of research funding.

- The University of Wisconsin/American Family Children's Hospital received a three-year, $60,000 award from the Muscular Dystrophy Association (MDA) as one of the centers in the MDA's Care Center Network. The award will support the multidisciplinary MDA Care Center at American Family Children's Hospital, led by **Mary Schroth, MD**.

  Dr. Schroth, along with UW colleagues Mei Baker, MD, and Meredith Schultz, MD, and Children's Hospital of Wisconsin colleague Matthew Harmelink, MD, nominated spinal muscular atrophy (SMA) as a disease to be included in the Department of Health Services (DHS) Newborn Screening Program in December 2017. Their nomination was approved by the DHS Secretary's Advisory Committee on Newborn Screening in January 2018. The group is now awaiting approval to implement SMA screening statewide.

- In 2017, *U.S. News and World Report* ranked American Family Children's Hospital (AFCH) among the top 50 children's hospitals for pulmonology (https://www.uwhealthkids.org/news-and-events/american-family-childrens-hospital-ranks-among-us-news-world-report-best-hospitals/51058).

- **HuiChan Lai, PhD, RD,** continued to explore the impact of nutrition in lung function in infants with cystic fibrosis with Cystic Fibrosis Foundation Therapeutics award to correlate quantitative radiologic and lung function measurements. She and **Philip Farrell, MD, PhD,** were also awarded a Legacy of Angels Foundation grant to support the first whole genome sequencing project of infants enrolled in the FIRST study. (Read related story [link to YIR item on CF]).

- The UW Pediatric Pulmonary Center was the "host" center for the annual Maternal and Child Health Bureau Annual Pediatric Pulmonary center meeting in Washington, DC. The focus of this meeting was using social media in the recruitment and training of the next generation of pediatric pulmonary interdisciplinary professionals.

RECENT PUBLICATIONS

Farrell PM, White TB, Derichs N, Castellani C, Rosenstein BJ. Cystic fibrosis diagnostic challenges over 4 decades: Historical perspectives and lessons learned. J Pediatr. 2017 Feb;181S:S16-S26. doi: 10.1016/j.jpeds.2016.09.067. PubMed PMID: 28129808.

Farrell PM, White TB, Howenstine MS, Munck A, Parad RB, Rosenfeld M, Sommerburg O, Accurso FJ, Davies JC, **Rock MJ**, Sanders DB, Wilschanski M, Sermet-Gaudelus I, Blau H, Gartner S, McColley SA. Diagnosis of cystic fibrosis in screened populations. J Pediatr. 2017 Feb;181S:S33-S44.e2. doi: 10.1016/j.jpeds.2016.09.065. PubMed PMID: 28129810.

Farrell PM, White TB, Ren CL, Hempstead SE, Accurso F, Derichs N, Howenstine M, McColley SA, **Rock M**, Rosenfeld M, Sermet-Gaudelus I, Southern KW, Marshall BC, Sosnay PR. Diagnosis of cystic fibrosis: Consensus guidelines from the Cystic Fibrosis Foundation. J Pediatr. 2017 Feb;181S:S4-S15.e1. doi: 10.1016/j.jpeds.2016.09.064. PubMed PMID: 28129811.

Farrell PM, White TB. Introduction to "Cystic Fibrosis Foundation consensus guidelines for diagnosis of cystic fibrosis". J Pediatr. 2017 Feb;181S:S1-S3. doi: 10.1016/j.jpeds.2016.09.062. PubMed PMID: 28129807.

Finkel RS, Mercuri E, Meyer OH, Simonds AK, **Schroth MK**, Graham RJ, Kirschner J, Iannaccone ST, Crawford TO, Woods S, Muntoni F, Wirth B, Montes J, Main M, Mazzone ES, Vitale M, Snyder B, Quijano-Roy S, Bertini E, Davis RH, Qian Y, Sejersen T; SMA Care group. Diagnosis and management of spinal muscular atrophy: Part 2: Pulmonary and acute care; medications, supplements and immunizations; other organ systems; and ethics. Neuromuscul Disord. 2017 Nov 23. pii: S0960-8966(17)31290-7. doi: 10.1016/j.nmd.2017.11.004. [Epub ahead of print] PubMed PMID: 29305137. *

Gumina DL, Black CP, **Balasubramaniam V,** Winn VD, Baker CD. Umbilical cord blood circulating progenitor cells and endothelial colony-forming cells are decreased in preeclampsia. Reprod Sci. 2017 Jul;24(7):1088-1096. doi: 10.1177/1933719116678692. Epub 2016 Nov 23. PubMed PMID: 27879452.

Lenhart RL, Youlo S, **Schroth MK**, Noonan KJ, McCarthy J, Mann D, Hetzel S, Sund SA, Halanski MA. Radiographic and respiratory effects of growing rods in children with spinal muscular atrophy. J Pediatr Orthop. 2017 Dec;37(8):e500-e504. doi: 10.1097/BPO.0000000000000867. PubMed PMID: 27636914; PubMed Central PMCID: PMC5352553.

Mercuri E, Finkel RS, Muntoni F, Wirth B, Montes J, Main M, Mazzone ES, Vitale M, Snyder B, Quijano-Roy S, Bertini E, Davis RH, Meyer OH, Simonds AK, **Schroth MK**, Graham RJ, Kirschner J, Iannaccone ST, Crawford TO, Woods S, Qian Y, Sejersen T; SMA Care Group. Diagnosis and management of spinal muscular atrophy: Part 1: Recommendations for diagnosis, rehabilitation, orthopedic and nutritional care. Neuromuscul Disord. 2017 Nov 23. pii: S0960-8966(17)31284-1. doi: 10.1016/j.nmd.2017.11.005. [Epub ahead of print] PubMed PMID: 29290580. *

Sanders DB, Li Z, Zhao Q, **Farrell PM**. Poor recovery from a pulmonary exacerbation does not lead to accelerated FEV(1) decline. J Cyst Fibros. 2017 Jul 29. pii: S1569-1993(17)30818-4. doi: 10.1016/j.jcf.2017.07.003. [Epub ahead of print] PubMed PMID: 28765072; PubMed Central PMCID: PMC5788732. *

Sanders DB, Zhao Q, Li Z, **Farrell PM**. Poor recovery from cystic fibrosis pulmonary exacerbations is associated with poor long-term outcomes. Pediatr Pulmonol. 2017 Oct;52(10):1268-1275. doi: 10.1002/ppul.23765. Epub 2017 Sep 7. PubMed PMID: 28881091; PubMed Central PMCID: PMC5639928.

Schieffler DA, **Farrell PM**, Kahn MJ, Culbertson RA. The evolution of the medical school deanship: From patriarch to CEO to system dean. Perm J. 2017;21. pii: 16-069. doi: 10.7812/TPP/16-069. PubMed PMID: 28241915; PubMed Central PMCID: PMC5283786.

Sosnay PR, Salinas DB, White TB, Ren CL, **Farrell PM**, Raraigh KS, Girodon E, Castellani C. Applying cystic fibrosis transmembrane conductance regulator genetics and CFTR2 data to facilitate diagnoses. J Pediatr. 2017 Feb;181S:S27-S32.e1. doi: 10.1016/j.jpeds.2016.09.063. PubMed PMID: 28129809.

Sosnay PR, White TB, **Farrell PM**, Ren CL, Derichs N, Howenstine MS, Nick JA, De Boeck K. Diagnosis of cystic fibrosis in nonscreened populations. J Pediatr. 2017 Feb;181S:S52-S57.e2. doi: 10.1016/j.jpeds.2016.09.068. PubMed PMID: 28129813.

Wong SL, **Balasubramaniam V**. A healthier future requires policy informed by lifespan science. Pediatr Res. 2017 Feb;81(2):280-281. doi: 10.1038/pr.2016.243. Epub 2016 Nov 15. PubMed PMID: 27846198. **

**ePub only; no print citation available when report was compiled*
*** Publication had previously appeared in 2015 report as an ePub*

GRANT SUPPORT

Faculty/Role	Funding Agency	Title
Farrell, Philip M (PI)	Cystic Fibrosis Foundation	Parental reactions to potential policies on the disclosure of incidental results in newborn screening for cystic fibrosis
Farrell, Philip (co-PI) with Baker, Mei Wang (co-PI) and Lai, Huichuan J (co-I)	Legacy of Angels	Assessing the added value of whole genome sequencing in cystic fibrosis newborn screening
Farrell, Philip (co-PI) with Baker, Mei Wang (co-PI)	Legacy of Angels	A prospective study of newborn screening for cystic fibrosis using a novel IRT/next generation sequencing method
Lai, Huichuan J (PI) with Farrell, Philip M (co-I)	Cystic Fibrosis Foundation Therapeutics	Feeding infants right...from the start (FIRST) study
Lai, Huichuan J (PI) with Farrell, Philip M and Rock, Michael J (co-Is)	Cystic Fibrosis Foundation Therapeutics	Impact of early malnutrition on lung disease development in cystic fibrosis
Lai, Huichuan J (PI) with Farrell, Philip M and Rock, Michael J (co-Is)	Cystic Fibrosis Foundation Therapeutics	Early childhood diet, growth, gut microbiome and lung health in cystic fibrosis
Lai, Huichuan J (PI) with Farrell, Philip M and Rock, Michael J (co-Is)	DHHS, PHS, National Institutes of Health	Early childhood diet, growth, gut microbiome and lung health in cystic fibrosis
Lai, Huichuan J (PI) with Farrell, Philip M (co-I)	DHHS, PHS, National Institutes of Health	Newborn screening, malnutrition and lung disease in children with cystic fibrosis
Lai, Huichuan J (PI)	Emory University	Method development for survival dynamic regression in chronic disease research

Lai, Huichuan J (PI)	Wisconsin Alumni Research Foundation	Travel grant
Matthews, Camilla B (co-I) with Jones, Stephanie (PI)	NIH/NICHD	Local sleep loss and neurobehavioral function in pediatric sleep-disordered breathing: a high density EEG investigation
Rock, Michael J (PI)	Children's Hospital and Regional Medical Center	EPIC-002
Rock, Michael J (PI)	Cystic Fibrosis Foundation	CF center
Rock, Michael J (PI)	Cystic Fibrosis Foundation	CF Center, FY17
Rock, Michael J (PI)	Cystic Fibrosis Foundation	CFF clinical fellowship (C. Barreda)
Rock, Michael J (PI)	Cystic Fibrosis Foundation Therapeutics	Additional research coordinator (ARC) award
Rock, Michael J (PI)	Cystic Fibrosis Foundation Therapeutics	CF therapeutic network CY17
Rock, Michael J (PI)	Legacy of Angels	A multi-center study of a new method of sweat testing: the CF quantum sweat test
Rock, Michael J (PI)	Seattle Children's Hospital	AquADEKs-2-IP-12
Rock, Michael J (PI)	Seattle Children's Hospital	CFFC-OB-11
Rock, Michael J (PI)	Seattle Children's Hospital	Optimizing treatment for early pseudomonas aeruginosa infection in cystic fibrosis: the OPTIMIZE multicenter randomized trial
Rock, Michael J (PI)	Seattle Children's Hospital	SHIP (saline hypertonic in preschoolers) study - planning grant
Rock, Michael J (PI)	Vertex Pharmaceuticals Inc	A phase 3, 2-arm, open-label study to evaluate the safety and pharmacodynamics of long-term ivacaftor treatment in subjects with cystic fibrosis who are less than 24 months of age at treatment initiation and have a CFTR gating mutation
Rock, Michael J (PI)	Vertex Pharmaceuticals Inc	A phase 3, randomized, double-blind, placebo-controlled, crossover study to evaluate the efficacy and safety of Ivacaftor and VX-661 in combination with Ivacaftor in subjects aged 12 years and older with cystic fibrosis, heterozygous for the F508del-CFTR mutation, and a second allele with a CFTR mutation predicted to have residual function
Rock, Michael J (PI)	Vertex Pharmaceuticals Inc	A phase 3, rollover study to evaluate the safety and efficacy of long-term treatment with Lumacaftor in combination with Ivacaftor in subjects aged 6 years and older with cystic fibrosis, homozygous for the F508del-CFTR mutation
Rock, Michael J (PI)	Vertex Pharmaceuticals Inc	A phase III, 2-part, open-label study to evaluate the safety, pharmacokinetics,

		and pharmacodynamics of ivacaftor in subjects with cystic fibrosis who are less than 24 months of age at treatment initiation and have a CFTR gating mutation
Rock, Michael J (PI)	Vertex Pharmaceuticals Inc	A phase III, double-blind, placebo-controlled, parallel-group study to evaluate the efficacy and safety of lumacaftor in combination with ivacaftor in subjects aged 6-11 years with cystic fibrosis, homozygous for the F508del-CFTR mutation
Rock, Michael J (PI)	Wisconsin Dept of Health Services	Congenital disorders
Schroth, Mary K (PI)	DHHS, PHS, Health Resources and Services Administration	MCHB University of Wisconsin pediatric pulmonary center
Schroth, Mary K (PI) with Matthews Camilla B and Rock Michael J (faculty)	DHHS, PHS, Health Resources and Services Administration	Pediatric Pulmonary Center (PPC) Training Grant
Schroth, Mary K (PI)	Muscular Dystrophy Association, Inc	MDA-sponsored clinic