

Stanford



Alejandro Dorenbaum

Clinical Professor, Pediatrics - Immunology and Allergy

CLINICAL OFFICE (PRIMARY)

- **Stanford Medicine Children's Health Specialty Services**
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Bio

CLINICAL FOCUS

- Pediatric Allergy/Immun
- Pediatric Allergy and Immunology

ACADEMIC APPOINTMENTS

- Clinical Professor, Pediatrics - Immunology and Allergy

ADMINISTRATIVE APPOINTMENTS

- Clinical Assistant Professor, Stanford University School of Medicine, (2000-2004)
- Clinical Associate Professor, Stanford University School of Medicine, (2004- present)

HONORS AND AWARDS

- Schering Travel Grant Award, 47th Annual Meeting of the AAAAI (1991)
- Schering Travel Grant Award, 48th Annual Meeting of the AAAAI (1992)
- Excellence in Teaching Award, Department of Pediatrics, UCSF (1995)
- Excellence in Teaching Award, Department of Pediatrics, UCSF (1997)

PROFESSIONAL EDUCATION

- Residency: University of Texas Health Science Center (1990) TX
- Internship: University of Texas Health Science Center (1988) TX
- Fellowship: Baylor College of Medicine (1992) TX
- Board Certification: Allergy and Immunology, American Board of Allergy and Immunology (1997)
- Board Certification, American Board of Pediatrics , Pediatrics (1990)
- Medical Education: Universidad Autonoma Metropolitana (1986) Mexico

COMMUNITY AND INTERNATIONAL WORK

- New Anti-HIV Medications: A Look at Nevirapine
- National Youth Leadership Forum on Medicine
- Bring Your Daughter to Work Program, UCSF
- Pediatric HIV Infection, from Diagnosis to Chronic Care, San Francisco
- Introduction to the Human Immune System, San Francisco
- HIV Weekly Update
- Is Pediatric HIV Increasing in Our Community?, Temple Beth El, San Francisco
- Can Infants Clear the HIV Infection from Their Blood?, San Francisco
- Pediatric Disease Prevention, radio broadcast, San Francisco
- Immunodeficiency in Children, radio broadcast, San Francisco
- Hemophilia Camp

Teaching

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

- Immunology (Phd Program)

Publications

PUBLICATIONS

- **Safety, tolerability, and efficacy of maralixibat in adults with primary sclerosing cholangitis: Open-label pilot study.** *Hepatology communications*
Bowlus, C. L., Eksteen, B., Cheung, A. C., Thorburn, D., Moylan, C. A., Pockros, P. J., Forman, L. M., Dorenbaum, A., Hirschfield, G. M., Kennedy, C., Jaecklin, T., McKibben, A., Chien, et al
2023; 7 (6)
- **Maralixibat for the treatment of PFIC: Long-term, IBAT inhibition in an open-label, Phase 2 study.** *Hepatology communications*
Loomes, K. M., Squires, R. H., Kelly, D., Rajwal, S., Soufi, N., Lachaux, A., Jankowska, I., Mack, C., Setchell, K. D., Karthikeyan, P., Kennedy, C., Dorenbaum, A., Desai, et al
2022
- **Efficacy and safety of maralixibat treatment in patients with Alagille syndrome and cholestatic pruritus (ICONIC): a randomised phase 2 study.** *Lancet (London, England)*
Gonzales, E., Hardikar, W., Stormon, M., Baker, A., Hierro, L., Gliwicz, D., Lacaille, F., Lachaux, A., Sturm, E., Setchell, K. D., Kennedy, C., Dorenbaum, A., Steinmetz, et al
2021; 398 (10311): 1581-1592
- **Efficacy and safety of maralixibat treatment in patients with Alagille syndrome and cholestatic pruritus (ICONIC): a randomised phase 2 study** *LANCET*
Gonzales, E., Hardikar, W., Stormon, M., Baker, A., Hierro, L., Gliwicz, D., Lacaille, F., Lachaux, A., Sturm, E., Setchell, K. R., Kennedy, C., Dorenbaum, A., Steinmetz, et al
2021; 398 (10311): 1581-1592
- **DURABILITY OF TREATMENT EFFECT WITH LONG-TERM MARALIXIBAT IN CHILDREN WITH ALAGILLE SYNDROME: 4-YEAR SAFETY AND EFFICACY RESULTS FROM THE ICONIC STUDY**
Gonzales, E. M., Sturm, E., Stormon, M., Sokal, E. M., Hardikar, W., Lacaille, F., Gliwicz-Miedzinska, D., Hierro, L., Jaecklin, T., Vig, P., Desai, N. K., Dorenbaum, A., Kennedy, et al
WILEY.2019: 1479A
- **SAFETY AND EFFICACY OF MARALIXIBAT IN PATIENTS WITH PRIMARY SCLEROSING CHOLANGITIS: AN OPEN-LABEL PROOF-OF-CONCEPT STUDY**
Bowlus, C. L., Eksteen, B., Cheung, A., Thorburn, D., Moylan, C. A., Pockros, P. J., Forman, L., Dorenbaum, A., Hirschfield, G., Kennedy, C., Gu, J., Apostol, G., Vig, et al

WILEY.2019: 764A–765A

● **Phase 2 open-label study with a placebo-controlled drug withdrawal period of the apical sodium-dependent bile acid transporter inhibitor maralixibat in children with Alagille Syndrome: 48-week interim efficacy analysis**

Gonzales, E., Sturm, E., Stormon, M., Sokal, E., Hardikar, W., Lacaille, F., Gliwicz, D., Hierro, L., Jaecklin, T., Gu, J., Desai, N. K., Dorenbaum, A., Kennedy, et al

ELSEVIER SCIENCE BV.2019: E119–E120

● **A Randomized, Controlled, Phase 2 Study of Maralixibat in the Treatment of Itching Associated With Primary Biliary Cholangitis HEPATOLOGY COMMUNICATIONS**

Mayo, M. J., Pockros, P. J., Jones, D., Bowlus, C. L., Levy, C., Patanwala, I., Bacon, B., Luketic, V., Vuppulanchi, R., Medendorp, S., Dorenbaum, A., Kennedy, C., Novak, et al

2019; 3 (3): 365–81

● **Development of a Novel Tool to Assess the Impact of Itching in Pediatric Cholestasis PATIENT-PATIENT CENTERED OUTCOMES RESEARCH**

Kamath, B. M., Abetz-Webb, L., Kennedy, C., Hepburn, B., Gauthier, M., Johnson, N., Medendorp, S., Dorenbaum, A., Todorova, L., Shneider, B. L. 2018; 11 (1): 69–82

● **CLARITY: A PHASE 2, RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED STUDY OF LOPIXIBAT CHLORIDE (FORMERLY LUM001), A NOVEL APICAL SODIUM-DEPENDENT BILE ACID TRANSPORTER INHIBITOR, IN THE TREATMENT OF PRIMARY BILIARY CIRRHOsis ASSOCIATED WITH ITCHING**

Mayo, M. J., Pockros, P., Jones, D., Bowlus, C., Levy, C., Patanwala, I., Bacon, B., Luketic, V., Vuppulanchi, R., Medendorp, S., Dorenbaum, A., Kennedy, C., Novak, et al

ELSEVIER SCIENCE BV.2016: S197

● **A Recombinant Antibody to Siglec-8 Shows Selective ADCC Activity Against Mast Cells from Systemic Mastocytosis Patients**

Falahati, R., Bright, J., Dorenbaum, A., Bebbington, C., Tomasevic, N., Lidke, D., George, T. I., Gotlib, J. AMER SOC HEMATOLOGY.2015

● **Impact of asthma exacerbations and asthma triggers on asthma-related quality of life in patients with severe or difficult-to-treat asthma. The journal of allergy and clinical immunology. In practice**

Luskin, A. T., Chipps, B. E., Rasoulian, L., Miller, D. P., Haselkorn, T., Dorenbaum, A. 2014; 2 (5): 544-52 e1 2

● **Single-dose, subcutaneous recombinant phenylalanine ammonia lyase conjugated with polyethylene glycol in adult patients with phenylketonuria: an open-label, multicentre, phase 1 dose-escalation trial LANCET**

Longo, N., Harding, C. O., Burton, B. K., Grange, D. K., Vockley, J., Wasserstein, M., Rice, G. M., Dorenbaum, A., Neuenburg, J. K., Musson, D. G., Gu, Z., Sile, S. 2014; 384 (9937): 37-44

● **Phenotypes determined by cluster analysis in severe or difficult-to-treat asthma JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY**

Schatz, M., Hsu, J. Y., Zeiger, R. S., Chen, W., Dorenbaum, A., Chipps, B. E., Haselkorn, T. 2014; 133 (6): 1549-1556

● **Assessment of asthma control and asthma exacerbations in the epidemiology and natural history of asthma: outcomes and treatment regimens (TENOR) observational cohort. Current respiratory care reports**

Chipps, B. E., Zeiger, R. S., Dorenbaum, A., Borish, L., Wenzel, S. E., Miller, D. P., Hayden, M. L., Bleeker, E. R., Simons, F. E., Szeffler, S. J., Weiss, S. T., Haselkorn, T. 2012; 1 (4): 259-269

● **Chaperone-like therapy with tetrahydrobiopterin in clinical trials for phenylketonuria: is genotype a predictor of response? JIMD reports**

Sarkissian, C. N., Gamez, A., Scott, P., Dauvillier, J., Dorenbaum, A., Scrivener, C. R., Stevens, R. C. 2012; 5: 59-70

● **Safety of extended treatment with sapropterin dihydrochloride in patients with phenylketonuria: Results of a phase 3b study MOLECULAR GENETICS AND METABOLISM**

Burton, B. K., Nowacka, M., Hennermann, J. B., Lipson, M., Grange, D. K., Chakrapani, A., Trefz, F., Dorenbaum, A., Imperiale, M., Kim, S. S., Fernhoff, P. M. 2011; 103 (4): 315-322

● **Relative Bioavailability of Sapropterin From Intact and Dissolved Sapropterin Dihydrochloride Tablets and the Effects of Food: A Randomized, Open-Label, Crossover Study in Healthy Adults CLINICAL THERAPEUTICS**

Musson, D. G., Kramer, W. G., Foehr, E. D., Bieberdorf, F. A., Hornfeldt, C. S., Kim, S. S., Dorenbaum, A.

2010; 32 (2): 338-346

- **Efficacy of Sapropterin Dihydrochloride in Increasing Phenylalanine Tolerance in Children with Phenylketonuria: A Phase III, Randomized, Double-Blind, Placebo-Controlled Study** *JOURNAL OF PEDIATRICS*

Trefz, F. K., Burton, B. K., Longo, N., Casanova, M. M., Gruskin, D. J., Dorenbaum, A., Kakkis, E. D., Crombez, E. A., Grange, D. K., Harmatz, P., Lipson, M. H., Milanowski, A., Randolph, et al
2009; 154 (5): 700-707

- **Safety and Efficacy of 22 Weeks of Treatment With Sapropterin Dihydrochloride in Patients With Phenylketonuria** *AMERICAN JOURNAL OF MEDICAL GENETICS PART A*

Lee, P., Treacy, E. P., Crombez, E., Wasserstein, M., Waber, L., Wolff, J., Wendel, U., Dorenbaum, A., Bebchuk, J., Christ-Schmidt, H., Seashore, M., Giovannini, M., Burton, et al
2008; 146A (22): 2851-2859

- **Pharmacokinetics of Sapropterin in Patients with Phenylketonuria** *CLINICAL PHARMACOKINETICS*

Feillet, F., Clarke, L., Meli, C., Lipson, M., Morris, A. A., Harmatz, P., Mould, D. R., Green, B., Dorenbaum, A., Giovannini, M., Foehr, E.
2008; 47 (12): 817-825

- **Pharmacokinetics of oral zidovudine administered during labour: a preliminary study** *HIV MEDICINE*

Mirochnick, M., Rodman, J. H., Robbins, B. L., Fridland, A., Gandia, J., Hitti, J., Bardeguez, A., Rathore, M. H., Garcia, A. G., Cababasay, M., Samson, P., Mofenson, L., Bryson, et al
2007; 8 (7): 451-456

- **The response of patients with phenylketonuria and elevated serum phenylalanine to treatment with oral sapropterin dihydrochloride (6R-tetrahydrobiopterin): a phase II, multicentre, open-label, screening study** *JOURNAL OF INHERITED METABOLIC DISEASE*

Burton, B. K., Grange, D. K., Milanowski, A., Vockley, G., Feillet, F., Crombez, E. A., Abadie, V., Harding, C. O., Cederbaum, S., Dobbelaere, D., Smith, A., Dorenbaum, A.
2007; 30 (5): 700-707

- **Phenylalanine blood levels and clinical outcomes in phenylketonuria: A systematic literature review and meta-analysis** *MOLECULAR GENETICS AND METABOLISM*

Waisbren, S. E., Noel, K., Fahrbach, K., Celli, C., Frame, D., Dorenbaum, A., Levy, H.
2007; 92 (1-2): 63-70

- **Efficacy of sapropterin dihydrochloride (tetrahydrobiopterin, 6R-BH4) for reduction of phenylalanine concentration in patients with phenylketonuria: a phase III randomised placebo-controlled study** *LANCET*

Levy, H. L., Milanowski, A., Chakrapani, A., Cleary, M., Lee, P., Trefz, F. K., Whitley, C. B., Feillet, F., Feigenbaum, A. S., Bebchuk, J. D., Christ-Schmidt, H., Dorenbaum, A.
2007; 370 (9586): 504-510

- **Efficacy and safety of heparinase I versus protamine in patients undergoing coronary artery bypass grafting with and without cardiopulmonary bypass** *ANESTHESIOLOGY*

Stafford-Smith, M., Lefrak, E. A., Qazi, A. G., Welsby, I. J., Barber, L., Hoeft, A., Dorenbaum, A., Mathias, J., Rochon, J. J., Newman, M. F.
2005; 103 (2): 229-240

- **The impact of race/ethnicity on mother-to-child HIV transmission in the united states in pediatric AIDS clinical trials group protocol 316 JAIDS**
JOURNAL OF ACQUIRED IMMUNE DEFICIENCY SYNDROMES

Cunningham, C. K., Balasubramanian, R., Delke, I., Maupin, R., Mofenson, L., Dorenbaum, A., Sullivan, J. L., Gonzalez-Garcia, A., Thorpe, E., Rathore, M., Gelber, R. D.
2004; 36 (3): 800-807

- **Maternal toxicity and pregnancy complications in human immunodeficiency virus-infected women receiving antiretroviral therapy: PACTG 316 10th Conference on Retroviruses and Opportunistic Infections**

Watts, D. H., Balasubramanian, R., Maupin, R. T., Delke, I., Dorenbaum, A., Fiore, S., Newell, M. L., Delfraissy, J. F., Gelber, R. D., Mofenson, L. M., Culnane, M., Cunningham, C. K.
MOSBY-ELSEVIER.2004: 506-16

- **Predose infant nevirapine concentration with the two-dose intrapartum neonatal nevirapine regimen: Association with timing of maternal intrapartum nevirapine dose** *9th Conference on Retroviruses and Opportunistic Infections*

Mirochnick, M., Dorenbaum, A., Blanchard, S., Cunningham, C. K., Gelber, R. D., Mofenson, L., Culnane, M., Sullivan, J. L.
LIPPINCOTT WILLIAMS & WILKINS.2003: 153-56

- When the time comes to talk about HIV: Factors associated with diagnostic disclosure and emotional distress in HIV-infected children JAIDS-JOURNAL OF ACQUIRED IMMUNE DEFICIENCY SYNDROMES
Lester, P., Chesney, M., Cooke, M., Weiss, R., Whalley, P., Perez, B., Glidden, D., Petru, A., Dorenbaum, A., Wara, D.
2002; 31 (3): 309-317
- Concentrations of protease inhibitors in cord blood after in utero exposure PEDIATRIC INFECTIOUS DISEASE JOURNAL
Mirochnick, M., Dorenbaum, A., Holland, D., Cunningham-Schrader, B., Cunningham, C., Gelber, R., Mofenson, L., Culnane, M., Connor, J., Sullivan, J. L.
2002; 21 (9): 835-838
- Envelope-based HIV vaccines SCIENCE
Donnelly, J. J., Barnett, S. W., Dorenbaum, A., Stamatatos, L.
2002; 297 (5585): 1277-1277
- Development of resistance mutations in women receiving standard antiretroviral therapy who received intrapartum nevirapine to prevent perinatal human immunodeficiency virus type 1 transmission: A substudy of Pediatric AIDS Clinical Trials Group protocol 316 8th Conference on Retroviruses and Opportunistic Infections
Cunningham, C. K., Chaix, M. L., Rekacewicz, C., Britto, P., Rouzioux, C., Gelber, R. D., Dorenbaum, A., Delfraissy, J. F., Bazin, B., Mofenson, L., Sullivan, J. L.
UNIV CHICAGO PRESS.2002: 181-88
- Two-dose intrapartum/newborn nevirapine and standard antiretroviral therapy to reduce perinatal HIV transmission - A randomized trial JAMA-JOURNAL OF THE AMERICAN MEDICAL ASSOCIATION
Dorenbaum, A., Cunningham, C. K., Gelber, R. D., Culnane, M., Mofenson, L., Britto, P., Rekacewicz, C., Newell, M. L., Delfraissy, J. F., Cunningham-Schrader, B., Mirochnick, M., Sullivan, J. L.
2002; 288 (2): 189-198
- When parents reject interventions to reduce postnatal human immunodeficiency virus transmission ARCHIVES OF PEDIATRICS & ADOLESCENT MEDICINE
Wolf, L. E., Lo, B., Beckerman, K. P., Dorenbaum, A., Kilpatrick, S., Weinrub, P. S.
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- HIV medical advances explored at retrovirus conference AIDS Policy Law
Dorenbaum A, Miserez AR
2001; 16 (10): 4
- Nevirapine - Pharmacokinetic considerations in children and pregnant women CLINICAL PHARMACOKINETICS
Mirochnick, M., Clarke, D. F., Dorenbaum, A.
2000; 39 (4): 281-293
- Cytokine profile of a long-term pediatric HIV survivor with hyper-IgE syndrome and a normal CD4(+) T-cell count JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY
Seroogy, C. M., Wara, D. W., Bluth, M. H., Dorenbaum, A., White, C., Durkin, H. G., Elder, M. E.
1999; 104 (5): 1045-1051
- Sleep disturbances in children with human immunodeficiency virus infection PEDIATRICS
Franck, L. S., Johnson, L. M., Lee, K., Hepner, C., Lambert, L., Passeri, M., Manio, E., Dorenbaum, A., Wara, D.
1999; 104 (5)
- Current applications for the use of nevirapine in HIV 1-infected pregnant women, infants and children Journal of HIV Therapy
Dorenbaum A, Mirochnick M
1999; 4 (3)
- Classification, diagnostic criteria, and treatment recommendations for orofacial manifestations in HIV-infected pediatric patients. Collaborative Workgroup on Oral Manifestations of Pediatric HIV Infection. journal of clinical pediatric dentistry
Ramos-Gomez, F. J., Flaitz, C., Catapano, P., Murray, P., Milnes, A. R., Dorenbaum, A.
1999; 23 (2): 85-96
- Phase I safety and pharmacokinetics study of micronized atovaquone in human immunodeficiency virus-infected infants and children ANTIMICROBIAL AGENTS AND CHEMOTHERAPY
Hughes, W., Dorenbaum, A., YOGEV, R., Beauchamp, B., Xu, J., McNamara, J., Moye, J., Purdue, L., Van Dyke, R., Rogers, M., Sadler, B.
1998; 42 (6): 1315-1318

- **Transplantation of CD34(+), CD3 haplocompatible peripheral blood stem cells (PBSC) for children with severe combined immunodeficiency disease (SCID).**
Cowan, M., Barley, F., DeSantes, K., Dorenbaum, A., Shames, R., Umetsu, D.
AMER SOC HEMATOLOGY.1997: 455-55
- **Transmission of HIV-1 in infants born to seropositive mothers: PCR-amplified proviral DNA detected by flow cytometric analysis of immunoreactive beads** *JOURNAL OF ACQUIRED IMMUNE DEFICIENCY SYNDROMES AND HUMAN RETROVIROLOGY*
Dorenbaum, A., Venkateswaran, K. S., Yang, G., Comeau, A. M., Wara, D., Vyas, G. N.
1997; 15 (1): 35-42
- **RECURRENT STAPHYLOCOCCUS-AUREUS RENAL ABSCESS IN A CHILD POSITIVE FOR THE HUMAN-IMMUNODEFICIENCY-VIRUS** *UROLOGY*
Brandeis, J. M., Baskin, L. S., Kogan, B. A., Wara, D., Dorenbaum, A.
1995; 46 (2): 246-248
- **RECOVERY OF MALASSEZIA-PACHYDERMATIS FROM 8 INFANTS IN A NEONATAL INTENSIVE-CARE NURSERY - CLINICAL AND LABORATORY FEATURES** *PEDIATRIC INFECTIOUS DISEASE JOURNAL*
LaRocco, M., Dorenbaum, A., Robinson, A., Pickering, L. K.
1988; 7 (6): 398-401
- **Case Report** *Infectious Disease Newsletter*
Dorenbaum A, Shearer WT, Abramson SL
1987; 6 (5): 38