



Alejandro Dorenbaum

Clinical Professor, Pediatrics - Immunology and Allergy

CLINICAL OFFICE (PRIMARY)

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

1100 Van Ness Ave 7th Fl

San Francisco, CA 94109

Tel (844) 733-2762 Fax (650) 725-7578

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., Vuppalanchi, 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., Vuppalanchi, 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., Rasouliyan, 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., Bleecker, E. R., Simons, F. E., Szefer, 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., Scriver, 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., Fahrback, K., Cella, 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., Weintrub, P. S.
2001; 155 (8): 927-933
- **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).**
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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.
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- **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