ERIC CROMBEZ, M.D.

CURRENT POSITION

Chief Medical Officer — November 2017 - Current
Gene Therapy and Inborn Errors of Metabolism
Ultragenyx

EMPLOYEMENT HISTORY

FDA Alternate Industry Representative — January 2020 - Current
Cellular, Tissue, and Gene Therapies Advisory Committee

Chief Medical Officer — December 2014 – November 2017
Dimension Therapeutics

Senior Medical Director — August 2010 – November 2014
Global Clinical Development
Shire

Medical Director — June 2007 – July 2010
Global Clinical Development
Shire

Assistant Professor, Department of Pediatrics, Division of Medical Genetics — July 2006 – May 2007
David Geffen School of Medicine at UCLA

Clinical Instructor, Department of Pediatrics, Division of Medical Genetics — July 2005 – June 2006
David Geffen School of Medicine at UCLA

EDUCATION AND TRAINING

University of Michigan, Ann Arbor, Michigan
Bachelor of Science, Biology — May 1994

University of Michigan Graduate School, Ann Arbor, Michigan
Cellular and Molecular Biology — June 1995

Wayne State University School of Medicine, Detroit, Michigan
Doctor of Medicine — June 2000

UCLA School of Medicine, Los Angeles, California
Residency, Departments of Pediatrics and Medical Genetics Combined Training Program — June 2005

UCLA School of Medicine, Los Angeles, California
Fellowship, Clinical Biochemical Genetics, Intercampus Genetics Training Program — June 2006

UCLA School of Medicine, Los Angeles, California
Fellowship in Translational Investigation

UCLA K30 program May 2006

LICENSURE AND ACCREDITATION

Medical Board of California: Certificate No. A78263
American Board of Medical Genetics, Clinical Genetics, 2005

PROFESSIONAL AFFILIATIONS

American Society of Human Genetics	2000 - present
American College of Medical Genetics	2003 - present
Society for Inherited Metabolic Disorder	2004 - present

HONORS AND AWARDS

O'Malley Foundation Fellowship in Urea Cycle Disorders	2004 and 2005
Society for Inherited Metabolic Disorders Travel Award for ICIEM 2006	2006
Los Angeles Super Doctors	2007

PUBLICATIONS

1. Metachromatic Leukodystrophy: An Assessment of Disease Burden. Eichler FS, Cox TM, **Crombez** E, Dali CÍ, Kohlschütter A. J Child Neurol. 2016 Nov;31(13):1457-1463.
2. Examination of the efficacy and safety of enzyme replacement therapy with velaglucerase alfa in Japanese Gaucher disease patients. Ida H, Tanaka A, Matsubayashi T, McCauley T, Wang N, Crombez E. J. Pediatr. Pract., 2015 78, 131-138.
3. Long-term velaglucerase alfa treatment in children with Gaucher disease type 1 naïve to enzyme replacement therapy or previously treated with imiglucerase. Smith L, Rhead W, Charrow J, Shankar SP, Bavdekar A, Longo N, Mardach R, Harmatz P, Hangartner T, Lee HM, **Crombez** E, Pastores GM. Mol Genet Metab. 2015 Jun 1
4. Seven-year safety and efficacy with velaglucerase alfa for treatment-naïve adult patients with type 1 Gaucher disease. Zimran A, Wang N, Ogg C, **Crombez** E, Cohn GM, Elstein D. Am J Hematol. 2015 Jul;90(7):577-83
5. Velaglucerase alfa (VPRIV) enzyme replacement therapy in patients with Gaucher disease: Long-term data from phase III clinical trials. Hughes DA, Gonzalez DE, Lukina EA, Mehta A, Kabra M, Elstein D, Kisinovsky I, Giraldo P, Bavdekar A, Hangartner TN, Wang N, **Crombez** E, Zimran A. Am J Hematol. 2015 Jul;90(7):584-91
6. Safety and efficacy results of switch from imiglucerase to velaglucerase alfa treatment in patients with type 1 Gaucher disease. Elstein D, Mehta A, Hughes DA, Giraldo P, Charrow J, Smith L, Shankar SP, Hangartner TN, Kunes Y, Wang N, **Crombez** E, Zimran A. Am J Hematol. 2015 Jul;90(7):592-7
7. Linear growth over 2 years of velaglucerase alfa therapy in children with type 1 Gaucher disease previously treated with imiglucerase. Zimarn A, Hughes D, Elstein D, Smith L, Harmatz P, Rhead W, Giraldo P, Mendelsohn N, Park CH, Zahrieh D, Crombez E. Bone Abstracts, 2013 2 P133.
8. Velaglucerase alfa enzyme replacement therapy compared with imiglucerase in patients with Gaucher disease. Ben Turkia H, Gonzalez DE, Barton NW, Zimran A, Kabra M, Lukina EA, Giraldo P, Kisinovsky I, Bavdekar A, Ben Dridi MF, Gupta N, Kishnani PS, Sureshkumar EK, Wang N, **Crombez** E, Bhirangi K, Mehta A. Am J Hematol. 2013 Mar;88(3):179-84.
9. Enzyme replacement therapy with velaglucerase alfa in Gaucher disease: Results from a randomized, double-blind, multinational, Phase 3 study. Gonzalez DE, Turkia HB, Lukina EA, Kisinovsky I, Dridi MF, Elstein D, Zahrieh D, **Crombez** E, Bhirangi K, Barton NW, Zimran A. Am J Hematol. 2013 Mar;88(3):166-71
10. Safety and efficacy of velaglucerase alfa in Gaucher disease type 1 patients previously treated with imiglucerase. Zimran A, Pastores GM, Tylki-Szymanska A, Hughes DA, Elstein D, Mardach R, Eng C, Smith L, Heisel-Kurth M, Charrow J, Harmatz P, Fernhoff P, Rhead W, Longo N, Giraldo P, Ruiz JA, Zahrieh D, **Crombez** E, Grabowski GA. Am J Hematol. 2013 Mar;88(3):172-8.

11. Efficacy of sapropterin dihydrochloride in increasing phenylalanine tolerance in children with phenylketonuria: a phase III, randomized, double-blind, placebo-controlled study. Trefz FK, Burton BK, Longo N, Casanova MM, Gruskin DJ, Dorenbaum A, Kakkis ED, **Crombez** EA, Grange DK, Harmatz P, Lipson MH, Milanowski A, Randolph LM, Vockley J, Whitley CB, Wolff JA, Bebchuk J, Christ-Schmidt H, Hennermann JB; Sapropterin Study Group. J Pediatr. 2009 May;154(5):700-7. doi: 10.1016/j.jpeds.2008.11.040. Epub 2009 Mar 4.
12. Clinical and molecular heterogeneity in patients with the cblD inborn error of cobalamin metabolism. Miousse IR, Watkins D, Coelho D, Rupar T, **Crombez** EA, Vilain E, Bernstein JA, Cowan T, Lee-Messer C, Enns GM, Fowler B, Rosenblatt DS. J Pediatr. 2009 Apr;154(4):551-6.
13. Arginase Deficiency. Cederbaum S, **Crombez** EA. In: Pagon RA, Adam MP, Bird TD, Dolan CR, Fong CT, Stephens K, editors. GeneReviews™ [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2013. 2004 Oct 21 [updated 2012 Feb 09].
14. Safety and efficacy of 22 weeks of treatment with sapropterin dihydrochloride in patients with phenylketonuria. Lee P, Treacy EP, **Crombez** E, Wasserstein M, Waber L, Wolff J, Wendel U, Dorenbaum A, Bebchuk J, Christ-Schmidt H, Seashore M, Giovannini M, Burton BK, Morris AA; Sapropterin Research Group. Am J Med Genet A. 2008 Nov 15;146A(22):2851-9.
15. Maternal glutaric acidemia, type I identified by newborn screening. **Crombez** EA, Cederbaum SD, Spector E, Chan E, Salazar D, Neidich J, Goodman S. Mol Genet Metab. 2008 May;94(1):132-4.
16. Burton BK, Grange DK, Milanowski A, Vockley G, Feillet F, **Crombez** EA, Abadie V, Harding CO, Cederbaum S, Dobbelaere D, Smith A, Dorenbaum A. The response of patients with phenylketonuria and elevated serum phenylalanine to treatment with oral sapropterin dihydrochloride: a Phase II, multicenter, open-label, screening study. Journal of Inherited Metabolic Disease. J Inherit Metab Dis. 2007 Oct;30(5):700-7. Epub 2007 Sep 12.
17. Expanded newborn screening identifies maternal primary carnitine deficiency. Schimmenti LA, **Crombez** EA, Schwahn BC, Heese BA, Wood TC, Schroer RJ, Bentler K, Cederbaum S, Sarafoglou K, McCann M, Rinaldo P, Matern D, di San Filippo CA, Pasquali M, Berry SA, Longo N.
18. Duplication of the Down syndrome critical region does not predict facial phenotype in a baby with a ring chromosome 21. **Crombez** EA, Dipple KM, Schimmenti LA, Rao N. Clin Dysmorphol. 2005 Oct;14(4):183-7.
19. Pitfalls in newborn screening. **Crombez** E, Koch R, Cederbaum S. J Pediatr. 2005 Jul;147(1):119-20.
20. Hyperargininemia due to liver arginase deficiency. **Crombez** EA, Cederbaum SD. Mol Genet Metab. 2005 Mar;84(3):243-51.
21. Isolation and characterization of the human and mouse homologues (SUPT4H and Supt4h) of the yeast SPT4 gene. Chiang PW, Wang SQ, Smithivas P, Song WJ, **Crombez** E, Akhtar A, Im R, Greenfield J, Ramamoorthy S, Van Keuren M, Blackburn CC, Tsai CH, Kurnit DM. Genomics. 1996 Jun 15;34(3):368-75.
22. Identification and analysis of the human and murine putative chromatin structure regulator SUPT6H and Supt6h. Chiang PW, Wang S, Smithivas P, Song WJ, Ramamoorthy S, Hillman J, Puett S, Van Keuren ML, **Crombez** E, Kumar A, Glover TW, Miller DE, Tsai CH, Blackburn CC, Chen XN, Sun Z, Cheng JF, Korenberg JR, Kurnit DM. Genomics. 1996 Jun 15;34(3):328-33.
23. Expressed sequence tags from the long arm of human chromosome 21. Chiang PW, Dzida G, Grumet J, Cheng JF, Song WJ, **Crombez** E, Van Keuren ML, Kurnit DM. Genomics. 1995 Sep 20;29(2):383-9.
24. Transcription patterns of sequences on human chromosome 21. Kurnit DM, Cheng J, Zhu Y, Van Keuren ML, Jiang Y, Pan Y, Whitley K, **Crombez** E. Cytogenet Cell Genet. 1995;71(2):203-6.
25. Construction of human embryonic cDNA libraries: HD, PKD1 and BRCA1 are transcribed widely during embryogenesis. Buraczynska MJ, Van Keuren ML, Buraczynska KM, Chang YS, **Crombez** E, Kurnit DM. Cytogenet Cell Genet. 1995;71(2):197-202.

CHAPTERS

1. **Crombez EA** and Cederbaum SD. Urea cycle disorders. In *Neurology: Basic and Clinical Neurosciencæs*. Schapira et al. eds. Mosby Elsevier, Philadelphia, 2007. Chapter 110. pp. 1469-1476.
2. Cederbaum S, **Crombez E**. Arginase Deficiency in: GeneReviews at GeneTests: Medical Genetics Information Resource. Copyright, University of Washington, Seattle. 1997-2007. Available at http://www.genetests.org.