Joint Meeting of the Pediatric Advisory Committee and the Pediatric Ethics Subcommittee
Thursday, May 18, 2017
FDA White Oak Great Room (Building 31)
10903 New Hampshire Avenue
Silver Spring, MD 20993

8:30 AM	Welcome and Introductions	Mark Hudak, MD Chair, Pediatric Advisory Committee (PAC)
8:35 AM	Opening Statement	Marieann R. Brill, MBA, RAC, MT(ASCP) Designated Federal Official, PAC Office of Pediatric Therapeutics (OPT), Office of the Commissioner (OC), FDA
8:39 AM	Opening Remarks and Review of The Agenda	Robert "Skip" Nelson, MD, PhD Deputy Director and Senior Pediatric Ethicist, OPT/OC/FDA
8:45 AM	Additional Safeguards for Children in Research and Protocol Review Under 21 CFR 50.54	Donna Snyder, MD Pediatric Ethicist, OPT/OC/FDA
9:16 AM	The "Essence" Clinical Trial: Protocol Design and Obstacles	Perry Shieh, MD, PhD Associate Professor & Director of the Neuromuscular Program, Department of Neurology, David Geffen School of Medicine, UCLA
10:04AM	UCLA IRB FDA Referral on the ESSENCE Trial for Duchenne Muscular Dystrophy	James McGough, MD Professor of Clinical Psychiatry, Semel Institute for Neuroscience and Human Behavior, and David Geffen School of Medicine, UCLA
10:34 AM	The Patient and Parent Perspective	Brett Bullers, Erin Bullers, and Nicholas Bullers
10:51 AM	Break	
11:10 AM	Open Public Hearing	
12:10 PM	Lunch	
1:20 PM	Sponsor Presentation	Genevieve Laforet, MD, PhD, Medical Director, Sarepta Therapeutics
1:54 PM	Presentation of Questions to the Committee	Robert "Skip" Nelson, MD, PhD Deputy Director, OPT/OC/FDA
2:20 PM	Committee Discussion and Vote	Mark Hudak, MD Chair, PAC
3:28 PM	Adjournment	Mark Hudak, MD Chair, PAC