FOOD AND DRUG ADMINISTRATION
Center for Biologics Evaluation and Research (CBER)

67th Meeting of the Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
October 12, 2017

FDA White Oak Conference Center, Room 1503
Silver Spring, Maryland

AGENDA

Topic: The committees will discuss Voretigene Neparvovec, Spark Therapeutics, BLA 125610, for the treatment of patients with vision loss due to confirmed biallelic RPE65 mutation-associated retinal dystrophy

Time	Presentation	Presenter
8:30 a.m.	Welcome and Introduction of Members	**Barry Byrne, M.D. Ph.D.** Acting Chair, CTGTAC
	Conflicts of Interest Statement	**Prabhakara Atreya, Ph.D.** Designated Federal Officer, CTGTAC
8:50 a.m.	FDA Introduction	**Wilson W. Bryan, M.D.** Director, Office of Tissues and Advanced Therapies (OTAT), CBER
	Sponsor Presentation(s)	
9:00 a.m.	Introduction	**Kathryn High, M.D.** President and Head of Res. & Dev
	Unmet Need	**Mark Pennessi, M.D. Ph.D.** Associate Prof. Ophthal. Genetics Oregon Health and Sci. University
	Efficacy	**Kathleen Reape, M.D.** Head, Clinical Res. & Dev
	Safety	**Deborah Kelley, M.D.** Head, Pharmacovigilance
	Clinical Perspective	**Albert Maguire, M.D.** Clin. Associate, Div. Ped. Ophthal. Children's Hospital of Philadelphia
	FDA Presentation	
10:00 a.m.	BLA125610 Voretigene Neparvovec Spark Therapeutics, Inc	**Yao-Yao Zhu, M.D., Ph.D.** Medical Officer, OTAT/CBER
10:45 a.m.	Break	
11:00 a.m.	Q & A	
11:15 a.m.	Open Public Hearing	
12:15 p.m.	Lunch	
1:15 p.m.	Q&A	
1:30 p.m.	Committee Discussion	
2:45 p.m.	Break	
3:00 p.m.	Committee Discussion	
5:00 p.m.	Adjourn Meeting	