DEVELOPING THERAPIES FOR PRIMARY MITOCHONDRIAL DISEASES: BRIDGING THE GAPS

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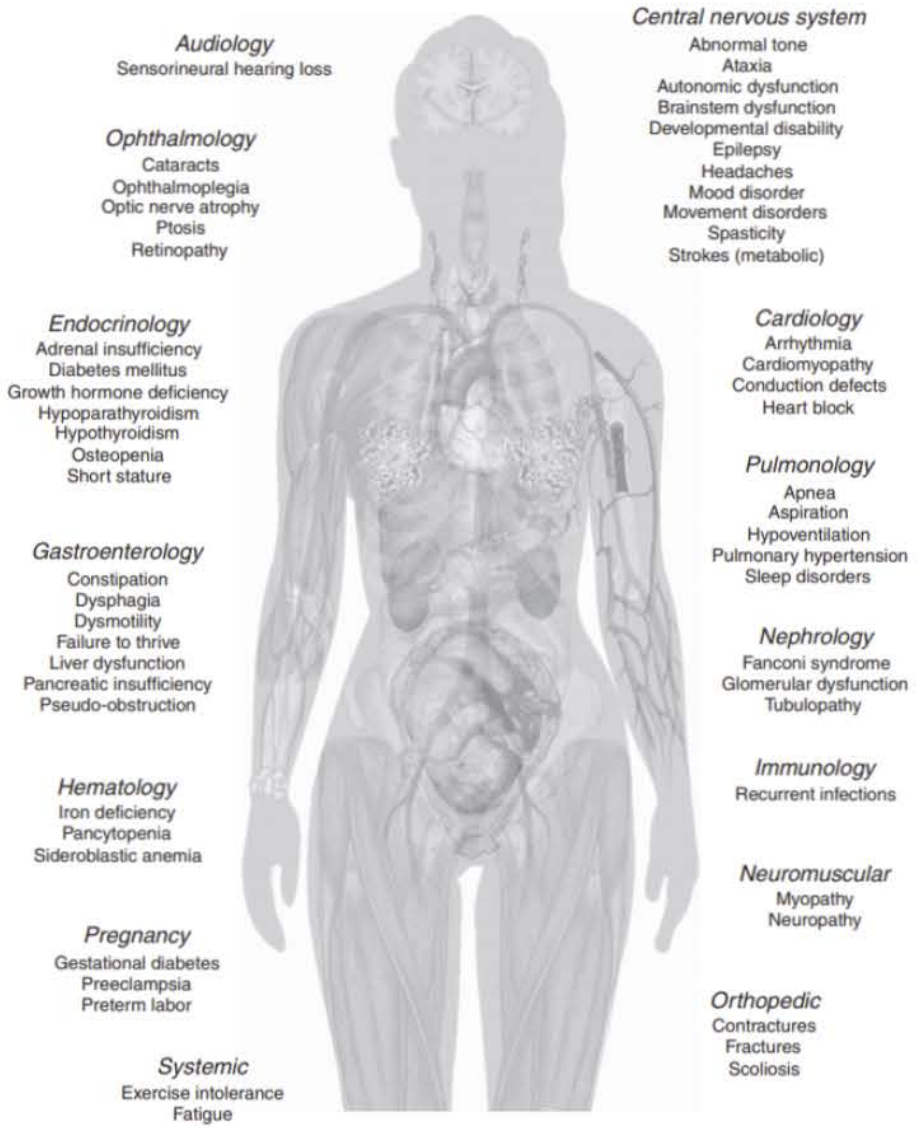
FDA White Oak Campus, The Great Room
September 6, 2019



Sumit Parikh et al: Patient care standards for primary mitochondrial disease: a consensus statement from the Mitochondrial Medicine Society, Genetics in Medicine, 2017

Developing Therapies for Primary Mitochondrial Diseases: Bridging the Gaps

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Agenda

Time	Topic	Speakers
8:00-8:05 am	Welcome/ Introduction	Dragos Roman, FDA
8:05-8:30 am	Integrating mitochondrial biology into designing drug development programs	Robert Naviaux, UCSD
8:30-9:00 am	Lessons learned from previous drug development programs	Jim Carr, Stealth Biotherapeutics Matthew Klein, BioElectron
9:00-9:45 am	Leveraging natural history data when designing clinical trials	-Michio Hirano, Columbia Univ -Philip Yeske, UMDF -Anita Zaidi, FDA
9:45-10:15 am	Patient population selection and considerations for pediatric patient enrollment in clinical trials	-Amel Karaa, Mass General Hosp -Melanie Bhatnagar, FDA
10:15-11:00 am	Panel discussion #1	
11:00-11:45	Open discussion/Q&A	
11:45-1:00 pm	Lunch	
1:00-1:45	Defining and assessing clinical benefit: regulatory, scientific, and patient perspectives	-Sophia Hufnagel, FDA -Bruce Cohen, Akron Childr Hosp -Phil Yeske, UMDF
1:45-2:15 pm	Panel discussion #2	
2:15-2:45pm	Clinical trial design and statistical considerations	-Frank Sasinowski, Hyman, Phelps & McNamara, P.C -Yan Wang, FDA
2:45-3:15 pm	Panel discussion #3	
3:15-3:45pm	Open discussion/Q&A	
3:45-4:00 pm	Summary/Next Steps	