

# Joint MDA-NINDS Workshop on Best Practices for Gene Therapy Programs

Meeting Organizers: Valerie Cwik, Amelie Gubitz, Jane Larkindale, John Porter, Hao Wang

Location: 5635 Fishers Lane, Rockville, MD 20852

Date: April 22, 2014

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- 8:00-8:15 am            **Introduction:** Story Landis (NINDS), Valerie Cwik (MDA) & Amelie Gubitz (NINDS)
- 8:15-9:00 am            **Keynote**  
Sailing to Ithaka: Journeys in Clinical Gene Therapy  
Katherine High (University of Pennsylvania)
- 9:00-10:30 am         **Session 1: Establishing Adequate Scientific Premise for Clinical Trials in Gene Therapy**  
  
Panel members: Jeffrey Chamberlain (University of Washington), Dongsheng Duan (University of Missouri), Brian Kaspar (Research Institute at Nationwide Children's Hospital), John Lincecum (ALS TDI), Fulvio Mavilio (Genethon), Glen Nuckolls (NIAMS), Marco Passini (Genzyme), Mercedes Serabian (FDA-CBER), Hansell Stedman (University of Pennsylvania), and Dan Tagle (NCATS)
- 10:30-10:45 am        **Break**
- 10:45-12:15 pm        **Session 2: Addressing Regulatory Process Issues**  
  
Panel members: Serge Braun (AFM), Robert Brown (University of Massachusetts), Wilson Bryan (FDA-CBER), Barry Byrne (University of Florida), Howard Federoff (Georgetown University), Katherine Klinger (Genzyme), Cheryl McDonald (NHLBI Gene Therapy Resource Program), and Jerry Mendell (Research Institute at Nationwide Children's Hospital)
- 12:15-1:15 pm         **Lunch (on your own)**
- 1:15-2:00 pm           **Case Study: EMA Approval of Glybera**  
James Wilson (University of Pennsylvania)
- 2:00-2:15 pm           **Break**
- 2:15-3:45 pm           **Session 3: IP and Commercialization of Gene Therapy Development**  
  
Panel members: Dale Berkley (Office of General Counsel, NIH Branch), James McLaughlin (Voyager Therapeutics), Bonnie Weiss McLeod (Cooley LLP), and Luk Vandenberghe (Harvard Medical School)
- 3:45-4:55 pm           **Discussion/Best Practices Recommendations**  
Howard Federoff (Georgetown University), Katherine High (University of Pennsylvania), Brian Kaspar (Research Institute at Nationwide Children's Hospital), Katherine Klinger (Genzyme), Fulvio Mavilio (Genethon), James McLaughlin (Voyager Therapeutics), Luk Vandenberghe (Harvard Medical School)
- 4:55-5:00 pm           **Closing Remarks:** John Porter (NINDS)