

**Supplemental Information**

**Next-generation strategies for gene-targeted  
therapies of central nervous system  
disorders: A workshop summary**

**Jill A. Morris, Chris H. Boshoff, Nina F. Schor, Ling M. Wong, Guangping Gao, and Beverly L. Davidson**

## **Appendix: Key Opinion Leaders (in alphabetical order)**

### **\*Co-chairs**

Rebecca Ahrens-Nicklas, M.D., Ph.D., The Children's Hospital of Philadelphia

Neil Aronin, M.D., University of Massachusetts Medical School

Aravind Asokan, Ph.D., Duke University School of Medicine

Fouad Atouf, Ph.D., U.S. Pharmacopeia

Douglas Ballon, Ph.D., Weill Cornell Medical College

Krystof Bankiewicz, M.D., The Ohio State University

Diana Bharucha-Goebel, M.D., NIH - NINDS & Children's National

Brian Bigger, Ph.D., University of Manchester

Ernesto Bongarzone, Ph.D., University of Illinois at Chicago

Carsten Bönnemann, M.D., NIH – NINDS

### **\*Chris H. Boshoff, Ph.D., NIH – NINDS**

Xandra Breakefield, Ph.D., Massachusetts General Hospital & Harvard Medical School

Ying Kai Chan, Ph.D., Ally Therapeutics

Theresa Chen, Ph.D., Food and Drug Administration

### **\*Beverly L. Davidson, Ph.D., University of Pennsylvania & The Children's Hospital of Philadelphia**

M. Jaysson Eicholtz, Scout Bio

Kevin Flanigan, M.D., Nationwide Children's Hospital

Omar Francone, Ph.D., Homology Medicines

Margie Frazier, Ph.D., MSW, Rare Disease Patient Advocacy and Consultation

### **\*Guangping Gao, Ph.D., University of Massachusetts Medical School**

Pedro Gonzalez-Alegre, M.D., Ph.D., University of Pennsylvania

Viviana Gradiaru, Ph.D., California Institute of Technology

Steven Gray, Ph.D., University of Texas Southwestern Medical Center

Heather Gray-Edwards, D.V.M., Ph.D., University of Massachusetts Medical School

Lyn Jakeman, Ph.D., NIH – NINDS

Petra Kaufmann, M.D., AveXis, INC.

Walter Koroshetz, M.D., NIH – NINDS

Anna Kwiolas, Ph.D., Food and Drug Administration

Timothy LaVaute, Ph.D., NIH – NINDS

Gerry Lipshutz, M.D., University of California, Los Angeles

Laura Mamounas, Ph.D., NIH – NINDS

Linda McGavern, Ph.D., NIH – NINDS

Joseph Menetski, Ph.D., Foundation for the NIH

Federico Mingozi, Ph.D., Spark Therapeutics

### **\*Jill A. Morris, Ph.D., NIH – NINDS**

Stephanie Morris, Ph.D., NIH - Office of Strategic Coordination

Avindra Nath, M.D., NIH – NINDS

Glen Nuckolls, Ph.D., NIH – NINDS

Anne Pariser, M.D., NIH – NCATS

Lauren Reoma, M.D., NIH – NINDS

Jim Richardson, Ph.D., U.S. Pharmacopeia

Becky Roof, Ph.D., NIH – NINDS

Richard Samulski, Ph.D., University of North Carolina at Chapel Hill  
Mark Sands, Ph.D., Washington University School of Medicine  
Nina Schor, M.D., Ph.D., NIH – NINDS  
Nita Seibel, M.D., NIH – NCI  
Miguel Sena-Esteves, Ph.D., University of Massachusetts Medical School  
Mario Skiadopoulos, Ph.D., NIH – NINDS  
Dolan Sondhi, Ph.D., Weill Cornell Medical College  
Greg Stewart, Ph.D., Axovant  
Edmund (Ned) Talley, Ph.D., NIH – NINDS  
Amir Tamiz, Ph.D., NIH – NINDS  
Cynthia Tiffet, M.D., Ph.D., NIH – NHGRI  
Bryan Traynor, M.D., Ph.D., NIH – NIA  
Daniel Urban, Ph.D., Food and Drug Administration  
Luk Vandenbergh, Ph.D., Harvard Medical School  
Charles Venditti, M.D., Ph.D., NIH – NHGRI  
Charles Vite, D.V.M., Ph.D., University of Pennsylvania  
Piotr Walczak, M.D., Ph.D., Johns Hopkins University  
Yael Weiss, M.D., Ph.D., Ulrogenyx Pharmaceutical  
John Wolfe, Ph.D., D.V.M., University of Pennsylvania & Children's Hospital Philadelphia  
Ling Wong, Ph.D., NIH – NINDS  
Clinton Wright, M.D., NIH – NINDS  
John Fraser Wright, Ph.D. Stanford University  
Timothy Yu, M.D., Ph.D., Boston Children's Hospital